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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 20-F

(Mark One)

☐ **REGISTRATION STATEMENT PURSUANT TO SECTION 12(b) OR (g) OF THE SECURITIES EXCHANGE ACT OF 1934**

OR

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended January 31, 2017

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to_____

OR

☐ **SHELL COMPANY PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

Date of event requiring this shell company report _____

Commission file number: 001-36866

Summit Therapeutics plc

(Exact name of Registrant as specified in its charter)

England and Wales
(Jurisdiction of incorporation or organization)

136a Eastern Avenue
Milton Park, Abingdon
Oxfordshire OX14 4SB
United Kingdom
(Address of principal executive offices)

Glyn Edwards, Chief Executive Officer
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(Name, Telephone, E-mail and/or Facsimile number and Address of Company Contact Person)

Securities registered or to be registered pursuant to Section 12(b) of the Act.

Title of each class	Name of each exchange on which registered
American Depositary Shares, each representing 5 Ordinary Shares, par value £0.01 per share	The NASDAQ Stock Market LLC

Securities registered or to be registered pursuant to Section 12(g) of the Act.
None

Securities for which there is a reporting obligation pursuant to Section 15(d) of the Act.
None

Indicate the number of outstanding shares of each of the issuer's classes of capital or common stock as of the close of the period covered by the annual report.

61,841,566 ordinary shares, par value £0.01 per share

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

If this report is an annual or transition report, indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934. Yes ☐ No ☒

Note—checking the box above will not relieve any registrant required to file reports pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934 from their obligations under those Sections.

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☐ No ☐ (not required)

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, or a non-accelerated filer. See definition of "accelerated filer and large accelerated filer" in Rule 12b-2 of the Exchange Act (check one):

Large accelerated filer ☐ Accelerated filer ☐ Non-accelerated filer ☒

Indicate by check mark which basis of accounting the registrant has used to prepared the financial statements included in this filing:

U.S. GAAP ☐ International Financial Reporting Standards as issued by the International Accounting Standards Board ☒ Other ☐

If "Other" has been checked in response to the previous question, indicate by check mark which financial statement item the registrant has elected to follow. Item 17 ☐ Item 18 ☐

If this is an annual report, indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

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GENERAL INFORMATION

In this Annual Report on Form 20-F, references to "Summit," "we," "us," and "our" or to the "company" refer to Summit Therapeutics plc and its consolidated subsidiaries, except where context otherwise requires.

The trademarks, trade names and service marks appearing in this Annual Report on Form 20-F are the property of their respective owners.

PRESENTATION OF FINANCIAL AND OTHER DATA

The consolidated financial statement data as of January 31, 2017 and 2016 and for the three years ended January 31, 2017, 2016 and 2015 have been derived from our consolidated financial statements, as presented at the end of this Annual Report, which have been prepared in accordance with International Financial Reporting Standards, or IFRS, as issued by the International Accounting Standards Board, or IASB, and as adopted by the European Union and audited in accordance with the standards of the Public Company Accounting Oversight Board (United States).

All references in this Annual Report to "$" are to U.S. dollars and all references to "£" are to pounds sterling. Solely for the convenience of the reader, unless otherwise indicated, all pounds sterling amounts as of and for the year ended January 31, 2017 have been translated into U.S. dollars at the rate at January 31, 2017, the last business day of our fiscal year ended January 31, 2017, of £1.00 to $1.2585. These translations should not be considered representations that any such amounts have been, could have been or could be converted into U.S. dollars at that or any other exchange rate as of that or any other date.

FORWARD-LOOKING STATEMENTS

This Annual Report contains forward-looking statements that involve substantial risks and uncertainties. All statements contained in this Annual Report, other than statements of historical fact, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. The forward-looking statements in this Annual Report include, among other things, statements about:

- the timing and conduct of our clinical trials of ezutromid (formerly SMT C1100) for the treatment of patients with Duchenne muscular dystrophy and ridinilazole (formerly SMT19969) for the treatment of patients with *Clostridium difficile* infection, including statements regarding the timing of initiation and completion of the clinical trials and the period during which the results of the clinical trials will become available;
- the timing of and our ability to obtain marketing approval of ezutromid and ridinilazole, and the ability of ezutromid and ridinilazole to meet existing or future regulatory standards;
- our plans to continue the research and development of the F3 formulation of ezutromid, the F6 formulation of ezutromid and future generation utrophin modulators that we are developing in collaboration with the University of Oxford and Sarepta Therapeutics, Inc., or Sarepta;
- the potential benefits and future operation of our collaboration with Sarepta;
- our plans with respect to possible future collaborations and partnering arrangements;
- our plans to pursue research and development of other future product candidates;

- the potential advantages of ezutromid and ridinilazole;
- the rate and degree of market acceptance and clinical utility of ezutromid and ridinilazole;
- our estimates regarding the potential market opportunity for ezutromid and ridinilazole;
- our sales, marketing and distribution capabilities and strategy;
- our ability to establish and maintain arrangements for manufacture of ezutromid and ridinilazole;
- our intellectual property position;
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing;
- the impact of government laws and regulations; and
- our competitive position.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report, particularly in the "Risk Factors" section in this Annual Report, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Annual Report and the documents that we have filed as exhibits to this Annual Report completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements.

PART I

Item 1: Identity of Directors, Senior Management and Advisers

Not applicable.

Item 2: Offer Statistics and Expected Timetable

Not applicable.

Item 3: Key Information

A. Selected Financial Data

The following table summarizes our consolidated financial data as of the dates and for the periods indicated. The consolidated financial statement data as of January 31, 2017 and 2016 and for the years ended January 31, 2017, 2016 and 2015 have been derived from our consolidated financial statements, as presented at the end of this Annual Report, which have been prepared in accordance with IFRS, as issued by the IASB, and as adopted by the European Union and audited in accordance with the standards of the Public Company Accounting Oversight Board (United States). The consolidated financial statement data as of January 31, 2015, 2014 and 2013 and for the years ended January 31, 2014 and 2013 have been derived from our consolidated financial statements, which are not presented herein, which have also been prepared in accordance with IFRS as issued by the IASB, and as adopted by the European Union and audited in accordance with the standards of the Public Company Accounting Oversight Board (United States).

Our consolidated financial statements are prepared and presented in pounds sterling, our presentation currency. Solely for the convenience of the reader, our consolidated financial statements as of and for the year ended January 31, 2017 have been translated into U.S. dollars at £1.00 to $1.2585 based on the foreign exchange rates published by the Federal Reserve Bank of New York for January 31, 2017. Such convenience translations should not be construed as a representation that the pound sterling amounts have been or could be converted into U.S. dollars at this or at any other rate of exchange, or at all.

Our historical results are not necessarily indicative of the results that may be expected in the future. The following selected consolidated financial data should be read in conjunction with our audited consolidated financial statements included at the end of this Annual Report and the related notes and Item 5, “Operating and Financial Review and Prospects” below.

Selected Consolidated Income Statement Data

	Year Ended January 31,					
	2017	2017	2016(1)	2015(1)	2014(1)	2013(1)
	(in thousands, except per share data)					
Revenue	$ 2,899	£ 2,304	£ —	£ —	£ —	£ —
Other operating income	90	72	1,281	1,888	1,526	1,389
Operating loss	(31,279)	(24,853)	(20,346)	(12,233)	(7,027)	(5,083)
Finance income	10	8	30	51	9	11
Finance cost	(1,085)	(862)	(2,879)	(499)	(385)	(401)
Income tax credit	5,457	4,336	3,058	1,297	607	341
Loss for the period	(26,897)	(21,371)	(20,137)	(11,384)	(6,796)	(5,132)
Basic and diluted loss per ordinary share from continuing operations	$ (0.44)	£ (0.35)	£ (0.34)	£ (0.29)	£ (0.33)	£ (0.32)
Weighted average number of shares outstanding (in thousands)	61,549	61,549	59,102	39,599	20,510	15,809

Selected Consolidated Balance Sheet Data

	2017	2017	As of January 31, 2016(1)	2015(1)	2014(1)	2013(1)
			(in thousands)			
Cash and cash equivalents	$ 35,316	£ 28,062	£ 16,304	£ 11,265	£ 2,030	£ 3,379
Working capital(2)	(7,076)	(5,621)	1,327	359	(816)	(587)
Total assets	47,304	37,587	25,057	19,396	7,295	4,377
Accumulated losses reserve	(92,836)	(73,767)	(52,396)	(32,259)	(47,166)	(40,370)
Total (deficit)/equity	$ (4,396)	£ (3,493)	£ 16,080	£ 12,962	£ 2,779	£ 1,571

(1) Financial information has been adjusted following the change in accounting policy regarding charitable funding arrangements. See "Operating and Financial Review and Prospects."
(2) We define working capital as prepayments and other receivables (including current tax receivables) less current liabilities.

Exchange Rate Information

The table below shows the period end, average, high and low exchange rates of U.S. dollars per pound sterling published by the Federal Reserve Bank of New York for the periods shown. The exchange rate on March 24, 2017 was £1.00 to $1.2491. The rates set forth below are provided solely for your convenience and may differ from the actual rates used in the preparation of our consolidated financial statements included in this Annual Report.

	Period End(1)	Average(2)	Low	High
		($ per pound sterling)		
Fiscal Year Ended January 31:				
2013	1.586	1.593	1.536	1.628
2014	1.645	1.572	1.484	1.661
2015	1.503	1.634	1.502	1.717
2016	1.418	1.518	1.417	1.588
2017	1.259	1.331	1.212	1.469
Month Ended:				
September 2016	1.302	1.314	1.296	1.343
October 2016	1.221	1.233	1.216	1.284
November 2016	1.248	1.243	1.222	1.255
December 2016	1.234	1.248	1.222	1.271
January 2017	1.259	1.237	1.212	1.262
February 2017	1.242	1.249	1.242	1.264
March 2017 (through March 24, 2017)	1.249	1.230	1.215	1.253

(1) In the event that the period end fell on a day for which data are not available, the exchange rate on the prior most recent business day is given.
(2) The average of the noon buying rate for pounds sterling on the last day of each full month during the relevant year or each business day during the relevant month indicated.

B. Capitalization and Indebtedness

Not applicable.

C. Reasons for the Offer and Use of Proceeds

Not applicable.

D. Risk Factors

Our business has significant risks. You should consider carefully the risks described below, together with the other information contained in this Annual Report, including our financial statements and the related notes. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception. We expect to incur losses for at least the next several years and may never generate profits from operations or maintain profitability.

Since inception, we have incurred significant operating losses. Our net loss was approximately £21.4 million for the year ended January 31, 2017, £20.1 million for the year ended January 31, 2016 (as adjusted) and £11.4 million for the year ended January 31, 2015 (as adjusted). As of January 31, 2017, we had an accumulated deficit of £73.8 million. To date, we have financed our operations primarily through issuances of our ordinary shares and American Depositary Shares, or ADSs, payments to us under our license and collaboration agreement with Sarepta Therapeutics, Inc., or Sarepta, and development funding and other assistance from government entities, philanthropic, non-government and not for profit organizations and patient advocacy groups for our product candidates. We have devoted substantially all of our efforts to research and development, including clinical trials. We have not completed development of any drugs. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially in connection with conducting clinical trials for our lead product candidates, ezutromid (formerly SMT C1100) for the treatment of patients with Duchenne muscular dystrophy, or DMD, and ridinilazole (formerly SMT19969) for the treatment of patients with *Clostridium difficile* infection, or CDI, and seeking marketing approval for ezutromid and ridinilazole in the United States and the European Union, as well as other geographies. In addition, if we obtain marketing approval of ezutromid in the United States or other jurisdictions where we retain commercial rights, or ridinilazole, we expect to incur significant sales, marketing, distribution and outsourced manufacturing expense, as well as ongoing research and development expenses.

In addition, our expenses will increase if and as we:

- continue the research and development of the F3 formulation of ezutromid, the F6 formulation of ezutromid and future generation modulators that we are developing in collaboration with the University of Oxford and Sarepta;
- seek to identify and develop additional product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical development;
- ultimately establish a sales, marketing and distribution infrastructure in jurisdictions where we have retained commercialization rights and scale up external manufacturing capabilities to commercialize any product candidates for which we receive marketing approval;
- acquire or in-license other product candidates and technology;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- expand our physical presence; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

Our ability to generate profits from operations and remain profitable depends on our ability to successfully develop and commercialize drugs that generate significant revenue. Based on our current plans, we do not expect

to generate significant product sales revenue unless and until we obtain marketing approval for, and commercialize, ezutromid for the treatment of DMD or ridinilazole for the treatment of CDI. This will require us to be successful in a range of challenging activities, including:

- successfully initiating and completing clinical trials of ezutromid for the treatment of DMD and ridinilazole for the treatment of CDI;
- obtaining approval to market ezutromid for the treatment of DMD and ridinilazole for the treatment of CDI;
- protecting our rights to our intellectual property portfolio related to ezutromid and ridinilazole;
- contracting for the manufacture of clinical and commercial quantities of ezutromid and ridinilazole;
- negotiating and securing adequate reimbursement from third-party payors for ezutromid and ridinilazole; and
- establishing sales, marketing and distribution capabilities to effectively market and sell ezutromid in the United States and ridinilazole in the United States and the European Union, as well as other geographies.

We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to generate profits from operations. Even if we do generate profits from operations, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to generate profits from operations and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Our operations to date have been limited to organizing and staffing our company, developing and securing our technology, raising capital and undertaking preclinical studies and clinical trials of our product candidates. We have not yet demonstrated our ability to successfully complete development of any product candidates, obtain marketing approvals, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

Assuming we obtain marketing approval for any of our product candidates, we will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays and may not be successful in such a transition.

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we initiate and continue clinical trials of ezutromid for the treatment of DMD and ridinilazole for the treatment of CDI, continue our research activities and initiate preclinical programs for future product candidates. In addition, if we obtain marketing approval for ezutromid, in the United States or other jurisdictions where we retain commercial rights, ridinilazole or any of our future product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, we expect to continue to incur additional costs associated with operating as a public company in the United States in addition to in the United Kingdom. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

We believe that our existing cash and cash equivalents, including an anticipated $22.0 million payment for a near-term development milestone under the license and collaboration agreement with Sarepta, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements through December 31, 2018. In our DMD program, while we anticipate that these capital resources will allow us to obtain top-line data for our Phase 2 clinical trial of ezutromid, which we refer to as PhaseOut DMD, we do not expect these capital resources will be sufficient to complete our planned randomized, placebo controlled clinical trial of ezutromid. In addition, in our CDI program, while we also anticipate that these capital resources will allow us to conduct activities to prepare ridinilazole for our two, planned Phase 3 clinical trials, we do not expect to be able to complete these trials without significant additional capital. We have based the foregoing estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. This estimate assumes, among other things, that we do not obtain any additional funding through grants and clinical trial support or through new collaboration arrangements. Our future capital requirements will depend on many factors, including:

- the progress, costs and results of clinical trials of ezutromid for DMD and ridinilazole for CDI;
- the scope, progress, costs and results of preclinical development, laboratory testing and clinical trials for the F3 formulation of ezutromid, the F6 formulation of ezutromid and future generation modulators that we are developing in collaboration with the University of Oxford and Sarepta;
- the number and development requirements of other future product candidates that we pursue;
- the costs, timing and outcome of regulatory review of ezutromid, ridinilazole and our other future product candidates;
- the costs and timing of commercialization activities, including product sales, marketing, distribution and manufacturing, for any of our product candidates that receive marketing approval;
- subject to receipt of marketing approval, revenue received from commercial sales of ezutromid, ridinilazole or any of our other future product candidates;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against any intellectual property-related claims;
- the amounts we receive from Sarepta for development, regulatory and sales milestones and royalty payments under our license and collaboration agreement;
- our ability to establish and maintain collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the extent to which we acquire or invest in other businesses, products and technologies;
- the rate of the expansion of our physical presence; and
- the costs of operating as a public company in the United States and in the United Kingdom.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we are not planning to have commercially available for several years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. Additional financing may not be available to us on acceptable terms, or at all.

Raising additional capital may cause dilution to our investors, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, collaborations, strategic alliances, grants and clinical trial support

from government entities, philanthropic, non-government and not for profit organizations and patient advocacy groups, debt financings, and marketing, distribution or licensing arrangements. We do not have any committed external source of funds other than the amounts we are entitled to receive from Sarepta for development, regulatory and sales milestones and royalty payments under our license and collaboration agreement with Sarepta. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as an equity holder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends or other distributions.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Risks Related to the Development and Commercialization of our Product Candidates

We depend heavily on the success of our lead product candidates, ezutromid, which we are developing for the treatment of DMD, and ridinilazole, which we are developing for the treatment of CDI. All of our other programs are still in the discovery or candidate optimization stage. If we are unable to commercialize ezutromid and ridinilazole, or experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources in the development of ezutromid for DMD and ridinilazole for CDI, both of which are still in clinical development. Our ability to generate product revenues, which may not occur for several years, if ever, will depend heavily on the successful development and commercialization of ezutromid and ridinilazole. The success of each of these product candidates will depend on a number of factors, including the following:

- successful completion of clinical development;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity;
- protecting our rights in our intellectual property portfolio;
- establishing sales, marketing and distribution capabilities;
- launching commercial sales of ezutromid or ridinilazole, as applicable, if and when approved, whether alone or in collaboration with others;
- acceptance of ezutromid or ridinilazole, as applicable, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies; and
- maintaining a continued acceptable safety profile of ezutromid or ridinilazole, as applicable, following approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize ezutromid or ridinilazole, which would materially harm our business.

If clinical trials of our product candidates fail to demonstrate safety and efficacy to the satisfaction of the U.S. Food and Drug Administration, or the FDA, or the European Medicines Agency, or the EMA, or do not otherwise produce favorable results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of ezutromid, ridinilazole or any other product candidate.

In connection with obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. In particular, the small number of patients in our early clinical trials may make the results of these clinical trials less predictive of the outcome of later clinical trials. The design of a clinical trial can determine whether its results will support approval of a product, and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced or completed. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

For example, in 2009, we assigned certain technology relating to our DMD program to BioMarin DMD Regulator Limited, or BioMarin. BioMarin conducted a Phase 1 clinical trial of a prior formulation of ezutromid in 48 healthy adult volunteers. Subjects in this clinical trial achieved low systemic exposure of the drug, and there was variability in systemic exposure across subjects. Following this clinical trial of a prior formulation of ezutromid, BioMarin elected not to continue development of our assigned technology, citing pharmaceutical and pharmacokinetic challenges. In public statements, BioMarin indicated that it had concluded that the likelihood of achieving a therapeutic effect in DMD patients was highly unlikely. In 2010, BioMarin transferred the assets, and all commercialization rights, back to us. Our first Phase 1 clinical trial of ezutromid was conducted in healthy volunteers using a different formulation than the one evaluated by BioMarin. The results from this trial showed an improvement in plasma levels of ezutromid when administered orally with food. In our first Phase 1b clinical trial of ezutromid in DMD patients, patients had variable levels of ezutromid in the blood plasma following dosing, which we believe was potentially due to the impact of diet on absorption of ezutromid. In 2015, we reported top-line data from our second Phase 1b clinical trial of ezutromid in DMD patients, which we refer to as our Phase 1b modified diet trial, in which patients followed specific dietary guidance that recommended balanced proportions of fat, protein, and carbohydrates and dosing with a glass of whole milk. In our Phase 1b modified diet trial, while following specific dietary guidance, all of the patients in the trial achieved plasma levels of ezutromid that we believe may be sufficient to modulate the production of utrophin protein and possibly result in clinical benefit. In addition, in our Phase 1 clinical trial of the F6 formulation of ezutromid, the F6 formulation achieved a greater than six-fold increase in average maximum plasma levels in DMD patients compared to those achieved with the F3 formulation of ezutromid evaluated in our Phase 1 modified diet trial, and we believe such plasma levels are within the range necessary for a potential therapeutic effect. Nonetheless, while the results of our completed clinical trials to date suggest that diet may impact absorption of ezutromid, other disease related factors, such as abnormal gastrointestinal physiology, or other factors such as the level of activity of the liver enzyme CYP1A, may impact the absorption profile of DMD patients. Accordingly, it is possible that we will be unable to achieve plasma levels of ezutromid that are expected to bring therapeutic benefit in future clinical trials, and, in such a case, we will likely not be able to successfully complete the development of, obtain marketing approval for or commercialize this product candidate.

In addition, in our first Phase 1b clinical trial of ezutromid in DMD patients, patients experienced a statistically significant reduction in creatine kinase, or CK, and other enzyme markers of muscle damage following treatment with ezutromid. Although this was not a placebo controlled study and there may be other factors that influenced the results, we believed the lower levels of CK and the other enzymes compared to baseline potentially indicated

a reduction in muscle damage and may have been evidence of ezutromid activity. However, in our Phase 1b modified diet trial, we did not observe a change in the levels of CK when patients received ezutromid as compared to when patients received a placebo. Likewise, we may not observe changes in levels of CK or other enzyme markers of muscle damage in longer-term clinical trials.

If we are required to conduct additional clinical trials or other testing of ezutromid or ridinilazole or any other product candidate that we develop beyond those that we contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these clinical trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as we intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements or restrictions; or
- have the product removed from the market after obtaining marketing approval.

If we experience any of a number of possible unforeseen events in connection with our clinical trials, potential marketing approval or commercialization of our product candidates could be delayed or prevented.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- we may be unable to enroll a sufficient number of patients in our clinical trials to ensure adequate statistical power to detect any statistically significant treatment effects;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators, institutional review boards or independent ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks;
- regulators, institutional review boards or independent ethics committees may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and

- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, institutional review boards or independent ethics committees to suspend or terminate the clinical trials.

Our product development costs will increase if we experience delays in testing or marketing approvals. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

Because we are developing ezutromid for the treatment of a disease in which there is little clinical experience, there is increased risk that the outcome of our clinical trials of ezutromid will not be favorable.

There are currently only two approved therapies for the treatment of DMD that seek to alter the progression of the disease, one in the United States and one in Europe, and neither of which treat the entire DMD patient population. Data on the natural clinical progression of DMD remain limited despite the recent publication of data from natural history studies on DMD patients. This has resulted in limited clinical trial experience for the development of drugs to treat DMD. In particular, regulatory authorities in the United States and European Union have not issued definitive guidance as to how to measure and achieve efficacy. As a result, the design and conduct of clinical trials for DMD is subject to increased risk.

In the last few years, a test of the distance walked by a patient in six minutes, commonly referred to as the six minute walk test, has been used as an endpoint in several clinical trials of product candidates for patients with DMD. It is viewed by U.S. and European regulators as a key outcome measure for DMD trials. We may nonetheless experience setbacks with our clinical trials for ezutromid or the clinical trials for our future product candidates for DMD because of the limited clinical experience in this indication. For example, regulators have not yet established what change in the distance walked in the six minute walk test is required to be demonstrated in a clinical trial of a DMD therapy in order to signify a clinically meaningful result or obtain marketing approvals. As a result, we may not achieve the pre-specified endpoint with statistical significance in clinical trials of ezutromid or of our other future product candidates for DMD, which would decrease the chance of obtaining marketing approval for ezutromid or our other future product candidates for DMD.

Our focus on utrophin modulation as a potential treatment for DMD is unproven, and we do not know whether we will be able to develop any products of commercial value for this indication.

Our scientific approach for treating DMD focuses on the discovery and development of utrophin modulators. There is no marketed drug that relies on utrophin modulation whereby the production of utrophin is maintained to compensate for the lack of dystrophin for the treatment of DMD or any other indication. As a result, we may not be able to replicate the results of our preclinical studies in our clinical trials of ezutromid, and our focus on targeting utrophin modulation may not result in the discovery and development of commercially viable drugs that safely and effectively treat DMD or other muscle-wasting disorders.

Moreover, we have not yet identified the level of utrophin modulation and associated production of utrophin needed to provide a clinical benefit to DMD patients. In our two completed Phase 1b clinical trials of the F3 formulation of ezutromid and our Phase 1 clinical trial of the F6 formulation of ezutromid, we observed variable plasma levels of drug among patients. Patients dosed with the F6 formulation in our Phase 1 clinical trial all achieved plasma concentration levels exceeding the level that corresponded to a 50% increase in utrophin expression in our preclinical studies. Meanwhile, only a proportion of patients dosed with the F3 formulation of ezutromid in each of our Phase 1b trials had plasma concentrations exceeding this 50% level. We believe that all the patients may still have achieved plasma levels of ezutromid sufficient to modulate the production of utrophin to a lesser extent and possibly result in clinical benefit. This belief is based in part on the work of Professor Kay Davies and her research group at the University of Oxford, in which the continued expression of utrophin protein

in the transgenic lines of an *mdx* mouse, even at levels just above those in a normal *mdx* mouse, had a meaningful, positive effect on muscle performance. Nonetheless, we do not know whether utrophin modulation has been achieved with either formulation of ezturomid, and if it has, whether the level of utrophin modulation and production in fact resulted in a clinical benefit for these patients.

If we experience delays or difficulties in the enrollment of patients in our clinical trials, our receipt of necessary marketing approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates, including our ongoing and planned clinical trials of ezutromid and ridinilazole, if we are unable to locate and enroll a sufficient number of eligible patients to participate in these clinical trials. DMD is a rare disease with a relatively small patient population, which could result in slow enrollment of clinical trial participants. Because we expect that our current and planned clinical trials for DMD will be limited to boys in a specified age range and with a certain level of physical ability only, the number of patients eligible for our clinical trials is even smaller. Further, there are only a limited number of specialist physicians that treat DMD patients, and major clinical centers are concentrated in a few geographic regions. CDI is an acute infection that requires rapid diagnosis. For our clinical trials of ridinilazole, we need to identify potential patients, test them for CDI and enroll them on the clinical trial within a 24-hour period. In addition, our competitors in both DMD and CDI have ongoing clinical trials for product candidates that could be competitive with our product candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates.

Patient enrollment is affected by other factors, including:

- severity of the disease under investigation;
- eligibility criteria for the clinical trial in question;
- perceived risks and benefits of the product candidate under study;
- competition for patients, time and resources at clinical trials sites from other investigational therapies in clinical trials that target the same patient population;
- approval of other therapies to treat the indication that is being investigated in the clinical trial;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients in our ongoing and planned clinical trials of ezutromid and ridinilazole or any of our other clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

If serious adverse or inappropriate side effects are identified during the development of ezutromid or ridinilazole or any other product candidate, we may need to abandon or limit our development of that product candidate.

All of our product candidates are in clinical or preclinical development and their risk of failure is high. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive marketing approval. If our product candidates are associated with undesirable side effects or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective.

For example, although ezutromid has generally been well tolerated at all doses tested, one of the patients in our Phase 1 clinical trial of the F6 formulation of ezutromid exhibited changes in liver parameters in laboratory findings and withdrew from the trial, despite showing no clinical symptoms. The finding was classified as a serious adverse event.

Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause side effects or other safety issues that prevented further development of the compound. If we elect or are forced to suspend or terminate any clinical trial of our product candidates, the commercial prospects of such product candidate will be harmed and our ability to generate product revenues from such product candidate will be delayed or eliminated. Any of these occurrences could materially harm our business.

Even if ezutromid or ridinilazole or any other product candidate receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If ezutromid, ridinilazole or any of our other future product candidates receive marketing approval, such products may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenues or revenue from collaboration agreements, including our license and collaboration agreement with Sarepta, or any profits from operations. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages compared to alternative treatments or competitive products;
- the prevalence and severity of any side effects;
- the ability to offer our product candidates for sale at competitive prices, including in the case of ridinilazole, which we expect, if approved, will compete with vancomycin and metronidazole, both of which are available in generic form at low prices, and the antibiotic, fidaxomicin, and potentially other approaches to be used as an adjunctive therapy to antibiotics, such as the monoclonal antibody bezlotoxumab, or vaccines;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement;
- the timing of any such marketing approval in relation to other product approvals;
- support from patient advocacy groups; and
- any restrictions on concomitant use of other medications.

Our ability to negotiate, secure and maintain third-party coverage and reimbursement may be affected by political, economic and regulatory developments in the United States, the European Union and other jurisdictions. Governments continue to impose cost containment measures, and third-party payors are increasingly challenging prices charged for medicines and examining their cost effectiveness, in addition to their safety and efficacy. These and other similar developments could significantly limit the degree of market acceptance of ezutromid or ridinilazole or any of our other future product candidates that receive marketing approval.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be successful in commercializing ezutromid or ridinilazole or any other product candidate if and when such product candidates are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale or marketing of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a

sales and marketing organization or outsource these functions to third parties. If ezutromid receives marketing approval, we intend to commercialize it in the United States with our own focused, specialized sales force. In the European Union, Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States, we will rely on Sarepta to commercialize ezutromid pursuant to the license and collaboration agreement we entered into with Sarepta in October 2016. Sarepta also has an option to expand its commercial rights to include specified countries in Latin America, which means we may have to rely on Sarepta to commercialize ezutromid in these additional territories. We plan to evaluate the potential for utilizing additional collaboration, distribution and marketing arrangements with third parties to commercialize ezutromid in other jurisdictions where we retain commercialization rights. With respect to ridinilazole, we are currently exploring options to develop and commercialize this antibiotic. Our options include seeking third-party collaborators or securing non-dilutive funding from government entities and philanthropic, non-government and not for profit organizations. We may also determine to commercialize ridinilazole directly in the United States and Europe with our own specialized sales force. There are risks involved with establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales and marketing services, our product revenues or the profitability of these product revenues to us are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are acceptable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates and any products we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide.

There is currently no approved therapy for the treatment of DMD applicable to all DMD patients that seeks to alter the progression of the disease. Corticosteroids, such as prednisolone and deflazacort, are the current standard of care for DMD patients, although these are symptomatic treatments that do not address the underlying cause of DMD and their use can be associated with severe side-effects and concerns over weight gain.

A number of biopharmaceutical companies, including Sarepta, are developing treatments for DMD based on exon-skipping approaches. Sarepta received accelerated approval for eteplirsen (Exondys 51), which based on its

targeting of exon 51, has the potential to treat approximately 13% of DMD patients. We believe that there are three other exon-skiping therapies in clinical development that target exon 44, exon 45 and exon 53, and that addressing these three exons, along with exon 51, would treat in aggregate less than one-third of all DMD patients. PTC Therapeutics, Inc. is developing ataluren (Translarna™), which is a small molecule that enables formation of functional dystrophin in DMD patients with nonsense mutations. DMD caused by nonsense mutations affects approximately 13% of all DMD patients. The European Commission has granted conditional approval for ataluren in Europe, and PTC is commercializing ataluren in several European countries. A number of other companies are pursuing alternative therapeutic approaches for the treatment of DMD, including Pfizer, Inc. and Bristol-Myers Squibb Company, which are pursuing an approach based on muscle tissue growth through myostatin inhibition. Santhera Pharmaceuticals Holding AG is developing a treatment designed to delay the deterioration in respiratory function, and a number of other companies are developing gene therapy based approaches. For more information, see "Business—Competition" in this Annual Report. We believe that our approach of utrophin modulation has the potential to treat the entire population of DMD patients, unlike other DMD approaches that also seek to alter the progression of the disease but only address subsets of the total DMD population. We expect the price that we will charge for ezutromid, if approved, will reflect its status as an orphan drug that will be directed at a smaller population of patients.

Several pharmaceutical and biotechnology companies have established themselves in the market for the treatment of CDI, and several additional companies are developing products for the treatment of CDI. The current standard of care for CDI is treatment with the broad spectrum antibiotics vancomycin and metronidazole, both of which are available in generic form in the United States. Generic antibiotic therapies typically are sold at lower prices than branded antibiotics and generally are preferred by managed care providers of health services. The antibiotic fidaxomicin (Dificid™ in the United States and Dificlir™ in Europe), which is marketed in the United States by Cubist Pharmaceuticals, Inc., or Cubist, a wholly owned subsidiary of Merck & Co., Inc. and in Europe by Astellas Pharma Inc., was approved for treatment of CDI in the United States and the European Union. Other antibiotics in late-stage clinical trials include cadazolid, which was originally being developed by Actelion Pharmaceuticals Limited before global rights acquired by Johnson and Johnson in January 2017, and is currently in Phase 3 clinical development. Merck received approval from the FDA for bezlotoxumab (Zinplava™), a monoclonal antibody for the treatment of patients, in combination with an antibiotic, who have a high risk of disease recurrence. Other approaches in development for the treatment of CDI include vaccines and fecal biotherapy. For more information, see "Business—Competition" in this Annual Report.

Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are more effective, safer, have fewer or less severe side effects, are approved for broader indications or patient populations, or are more convenient or less expensive than any products that we develop and commercialize. Our competitors may also obtain marketing approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

We believe that many competitors are attempting to develop therapeutics for the target indications of our product candidates, including academic institutions, government agencies, public and private research organizations, large pharmaceutical companies and smaller more focused companies.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining approvals from regulatory authorities and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to or necessary for our programs.

Even if we are able to commercialize ezutromid, ridinilazole or any other product candidate that we develop, the product may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize ezutromid, ridinilazole or any other product candidate successfully also will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the E.U. and U.S. healthcare industries and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for ezutromid, ridinilazole or any other product that we commercialize and, if coverage and reimbursement are available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for ezutromid may be particularly difficult because of the higher prices typically associated with drugs directed at smaller populations of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement of a higher priced drug. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the applicable regulatory authority. Moreover, eligibility for coverage and reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, intellectual property, manufacture, sale and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. In the United States, third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. In the European Union, reference pricing systems and other measures may lead to cost containment and reduced prices. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, particularly the member states of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various E.U. member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product candidate to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third-party payors or authorities may lead to further pressure on prices or reimbursement levels within the country of publication and other countries. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be adversely affected.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- reduced resources of our management to pursue our business strategy;
- decreased demand for any product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to clinical trial participants or patients;
- loss of revenue;
- increased insurance costs; and
- the inability to commercialize any products that we may develop.

We have separate product liability insurance policies that cover each of our clinical trials. These policies each provide coverage of up to £5.0 million in the aggregate for clinical trials, or portions thereof, conducted in Europe and up to $5.0 million in the aggregate for clinical trials, or portions thereof, conducted in the United States. The insurance policies covering our clinical trials in the United States are also subject to a per claim deductible. The amount of insurance that we currently hold may not be adequate to cover all liabilities that we may incur. We will need to increase our insurance coverage when and if we begin commercializing ezutromid, ridinilazole or any other product candidate that receives marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes.

Our operations currently, and may in the future, involve the use of hazardous and flammable materials, including chemicals and medical and biological materials, and produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and wastes, we cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials or disposal of hazardous wastes, we could be held liable for any resulting damages, and any liability could exceed our resources.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We also maintain liability insurance for some of these risks, but our policy has a coverage limit of £5.0 million per occurrence.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

We may expend our limited resources to pursue a particular product candidate and fail to capitalize on product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on specific product candidates. As a result, we may forego or delay pursuit of opportunities with other product candidates that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates may not yield any commercially viable products.

We have based our research and development efforts for DMD on utrophin modulators, including the F3 formulation of ezutromid, the F6 formulation of ezutromid and our future generation utrophin modulators, and for CDI on ridinilazole. Notwithstanding our large investment to date and anticipated future expenditures in proprietary technologies that we use in the discovery of product candidates for DMD and CDI, we have not yet developed, and may never successfully develop, any marketed drugs using this approach. As a result of pursuing the development of product candidates using our proprietary technologies, we may fail to develop product candidates or address indications based on other scientific approaches that may offer greater commercial potential or for which there is a greater likelihood of success. Research programs to identify new product candidates require substantial technical, financial and human resources. These research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development.

If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

The United Kingdom's vote in favor of withdrawing from the European Union could lead to increased market volatility which could adversely impact the market price of our ordinary shares and ADSs and make it more difficult for us to do business in Europe.

On June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union (commonly referred to as "Brexit"). The withdrawal of the United Kingdom from the European Union will take effect either on the effective date of the withdrawal agreement or, in the absence of agreement, two years after the United Kingdom provides a notice of withdrawal pursuant to Article 50 of the E.U. Treaty, unless the European Council, in agreement with the United Kingdom, unanimously decides to extend this period. On

March 29, 2017, the U.K. Prime Minister formally delivered the notice of withdrawal. It appears likely that this withdrawal will involve a process of lengthy negotiations between the United Kingdom and European Union member states to determine the future terms of the United Kingdom's relationship with the European Union. This could lead to a period of considerable uncertainty particularly in relation to United Kingdom financial and banking markets as well as on the regulatory process in Europe. As a result of this uncertainty, financial markets could experience significant volatility which could adversely affect the market price of our ordinary shares and ADSs.

We may also face new regulatory costs and challenges that could have a material adverse effect on our operations. Depending on the terms of Brexit, the United Kingdom could lose the benefits of global trade agreements negotiated by the European Union on behalf of its members, which may result in increased trade barriers which could make our doing business in Europe more difficult. In addition, currency exchange rates in the pound sterling and the euro with respect to each other and the U.S. dollar have already been adversely affected by Brexit. Furthermore, at present, there are no indications of the effect Brexit will have on the pathway to obtaining marketing approval for any of our product candidates in the U.K., or what, if any, role the EMA may have in the approval process.

Risks Related to our Dependence on Third Parties

We will depend heavily on our license and collaboration arrangement with Sarepta for the success of the products in our utrophin modulator pipeline in the European Union and other geographies. If Sarepta terminates our license and collaboration agreement or is unable to meet its contractual obligations, it could negatively impact our business.

In October 2016, we entered into a license and collaboration agreement pursuant to which we granted exclusive rights to Sarepta to commercialize products in our utrophin modulator pipeline, or the licensed products, in the European Union (including the United Kingdom, irrespective of the timing of Brexit), Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States, or the licensed territories, with an option exercisable by Sarepta for commercialization rights to the licensed products in Central and South America.

Under the terms of the license and collaboration agreement, we are entitled to receive specified development, regulatory and sales milestone payments, as well as royalty payments. In addition, beginning in 2018, subject to certain exceptions and limitations, we will share all budgeted global research and development costs with Sarepta, with Sarepta responsible for 45.0% of these research and development costs for the licensed products. In addition, Sarepta will be solely responsible for all commercialization activities and associated costs, relating to licensed products in the licensed territories.

Unless earlier terminated, the license and collaboration agreement will expire on a licensed product-by-licensed product and country-by-country basis upon the expiration of the royalty term in such country for such licensed product. The license and collaboration agreement may be terminated by Sarepta upon six months' prior written notice in its entirety or on a licensed product-by-licensed product and country-by-country basis. Either party may, subject to a cure period, terminate the license and collaboration agreement in the event of the other party's uncured material breach. Sarepta may also terminate the license and collaboration agreement under specified circumstances relating to the safety or regulatory approvability of ezutromid.

If Sarepta were to terminate the license and collaboration agreement or fail to meet its contractual obligations, the assumption by us of all costs related to the development of products in our utrophin modulator pipeline and the establishment of a commercial infrastructure in the licensed territories would require substantial resources, financial and otherwise, and could result in us incurring greater expenses than the increase in revenues from our direct sales of the licensed products in the licensed territories. It could also cause a delay in the development of ezutromid. Seeking and obtaining a viable, alternative collaborator to partner on the development and commercialization of the licensed products may not be available on similar terms or at all.

We depend on collaborations with third parties for the development and commercialization of some of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.

We are evaluating our options to maximize the commercial opportunity for ridinilazole, including the relative merits of retaining commercialization rights for ourselves, seeking a third-party collaborator for ridinilazole and/or securing non-dilutive funding from government entities, philanthropic organizations and charities. In addition, we have entered into a license and collaboration agreement with Sarepta to commercialize ezutromid in Europe and other geographies, and, we plan to continue to evaluate the potential for utilizing collaboration, distribution and marketing arrangements with third parties to commercialize ezutromid in geographies where we retain commercialization rights, including the United States. Moreover, we may seek third-party collaborators for development and commercialization of any future product candidates.

Our likely future collaborators for any marketing, distribution, development, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. Under our license and collaboration agreement with Sarepta we have, and under any such arrangements we enter into with any third parties in the future we will likely have, limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements.

Our current collaboration poses, and any future collaboration likely will pose, numerous risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations and may not perform their obligations as expected;
- collaborators may deemphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus, including as a result of a sale or disposition of a business unit or development function, or available funding, or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to multiple products may not commit sufficient resources to the marketing and distribution of our product relative to other products;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborator and us as to the ownership of intellectual property arising during the collaboration;

- we may grant exclusive rights to our collaborators, which would prevent us from collaborating with others;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

For example, in 2009, we assigned certain technology relating to our DMD program to BioMarin. BioMarin conducted a Phase 1 clinical trial of a prior formulation of ezutromid in 48 healthy adult volunteers. In this clinical trial, subjects achieved low systemic exposure of the drug and there was variability of systemic exposure across subjects. Following this clinical trial of a prior formulation of ezutromid, BioMarin elected not to continue development of our assigned technology, citing pharmaceutical and pharmacokinetic challenges. In public statements, BioMarin indicated that it had concluded that the likelihood of achieving a therapeutic effect in DMD patients was highly unlikely. In 2010, BioMarin transferred the assets, and all commercialization rights, back to us.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

Use of third parties to manufacture our product candidates may increase the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not own or operate manufacturing facilities for the production of clinical or commercial supplies of our product candidates. We have limited personnel with experience in drug manufacturing and lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We currently rely on third parties for supply of the active pharmaceutical ingredients, or API, in our product candidates. Our strategy is to outsource all manufacturing of our product candidates and products to third parties.

We do not currently have any agreements with third-party manufacturers for the long-term clinical or commercial supply of any of our product candidates. We currently engage a single third-party manufacturer to provide clinical material of the API and fill and finish services for the final drug product of the F3 formulation of ezutromid that is being used in our Phase 2 clinical trial. We are engaged with a different drug product manufacturer to provide bulk drug product of the F6 formulation of ezutromid. A different third party manufacturer provides fill and finish services to supply the final drug product of the F6 formulation of ezutromid. We are engaged with a different third-party vendor to provide labelling, packaging and distribution services for the F3 and F6 formulations of ezutromid. We are engaged with another third-party manufacturer to provide clinical material of the API of ridinilazole with a different supplier responsible for fill and finish services to supply the final drug product for use in the planned Phase 3 clinical trials. We may be unable to conclude agreements for commercial supply with third-party manufacturers, or may be unable to do so on acceptable terms.

Even if we are able to establish and maintain arrangements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and

- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with current good manufacturing practice, or cGMP, regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

If the third parties that we engage to manufacture product for our preclinical tests and clinical trials should cease to continue to do so for any reason, we likely would experience delays in advancing these clinical trials while we identify and qualify replacement suppliers and we may be unable to obtain replacement supplies on terms that are favorable to us. In addition, if we are not able to obtain adequate supplies of our product candidates or the drug substances used to manufacture them, it will be more difficult for us to develop our product candidates and compete effectively. Our inability to obtain adequate supplies of ezutromid for preclinical tests and clinical trials may also impact Sarepta's ability to commercialize ezutromid and our second generation and future generation utrophin modulators. Under our license and collaboration agreement with Sarepta, we have agreed to use commercially reasonable efforts to supply to Sarepta active pharmaceutical ingredient, finished drug product and placebo for Sarepta to conduct research, development and commercialization activities.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability, and the ability of Sarepta and any other future collaborator, to develop product candidates and commercialize any products that receive marketing approval on a timely and competitive basis.

We rely on third parties to conduct our clinical trials and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such clinical trials.

We do not independently conduct clinical trials for our product candidates. We rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to perform this function. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the clinical trial. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practice, or GCP, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity of data and confidentiality of clinical trial participants are protected. The EMA imposes similar requirements on us for products that are the subject of clinical trials in European Union, including the United Kingdom.

We also are required to register ongoing clinical trials and post the results of completed clinical trials on a U.S. government-sponsored database, www.ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. In September 2016, the U.S. Department of Health and Human Services through the U.S. National Institutes of Health issued new regulations that expand the legal requirements for submitting registration and results information for clinical trials involving FDA-regulated drugs,

biologics and medical devices. The new rules require sponsors, among other things, to post results of clinical trials for unapproved products, including unfavorable results in clinical trials for unapproved uses of approved products. The EMA has also adopted transparency requirements that apply to clinical trials conducted in the European Union (EMA Policy/0070 on the publication of clinical data for medicinal products for human use, effective as of January 1, 2015). The EMA will implement this policy on the publication of clinical data in two phases. Phase 1 concerns the publication of clinical reports submitted to EMA as part of a marketing authorization application and through the centralized procedure. It entered into force on January 1, 2015. Phase 2 concerns the publication of individual patient data. The EMA will implement this phase at a later stage. This publication requirement for clinical reports may force us to disclose know-how relating to the design of clinical trials for our product candidates, which may harm our interests by disclosing valuable know-how to our competitors, which may be used to develop competing products to our drug candidates.

Furthermore, third parties that we rely on for our clinical development activities may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Our product development costs will increase if we experience delays in testing or obtaining marketing approvals.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

Our ability to identify and develop future generations of utrophin modulators depends on our strategic alliance with the University of Oxford. If we fail to maintain our current strategic relationship with the University of Oxford, our business prospects may be materially adversely affected.

We have formed a strategic alliance with the University of Oxford pursuant to which we acquired an exclusive option to license intellectual property that is generated as part of our research in utrophin modulation. The goal of our strategic alliance with the University of Oxford is to identify and develop future generations of utrophin modulators that will include new mechanisms that could complement ezutromid. We rely on this strategic alliance and the University of Oxford to help identify and develop future generations of utrophin modulators. The continuation of a good relationship with the University of Oxford is important to our discovery and research efforts in this area. If our relationship with the University of Oxford deteriorates, if the University of Oxford fails to devote sufficient resources to the strategic alliance or if the University of Oxford challenges our option to license any intellectual property generated as part of the strategic alliance, our business prospects could be materially adversely affected.

If we are not able to establish additional collaborations, we may have to alter our development and commercialization plans.

Our product development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate further with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by

regulatory authorities, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge; and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators and changes to the strategies of the combined company.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

If we fail to comply with our obligations in our funding arrangements with third parties, we could be required to repay the grant funding we have received or grant to these third parties rights under certain of our intellectual property.

We have received grant funding for some of our development programs from philanthropic, non-government and not for profit organizations and patient advocacy groups pursuant to agreements that impose development and commercialization diligence obligations on us. If we fail to comply with these obligations, in certain instances the applicable organization could require us to repay the grant funding we have received with interest or grant to the organization rights under certain of our intellectual property, which could materially adversely affect the value to us of product candidates covered by that intellectual property even if we are entitled to a share of any consideration received by such organization in connection with any subsequent development or commercialization of the product candidates.

Risks Related to our Intellectual Property

If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We seek to protect our proprietary position by filing patent applications in the United States, in Europe and in certain additional foreign jurisdictions related to our novel technologies and product candidates that are important to our business. This process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, if we license technology or product candidates from third parties in the future, these license agreements may not permit us to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering the licensed technology or product candidates. These agreements could also give our licensors the right to enforce the licensed patents without our involvement, or to decide not to enforce the patents at all. Therefore,

in these circumstances, these patents and applications may not be prosecuted or enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents, narrow the scope of our patent protection or make enforcement more difficult or uncertain.

The laws of foreign countries may not protect our patent rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does. In addition, for the foregoing reasons, we may not pursue or obtain patent protection in all major markets or may not obtain protection that enables us to prevent the entry of third parties into the market.

Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our U.S. patents or pending U.S. patent applications, or that we were the first to file for patent protection of such inventions outside the United States or, since March 16, 2013, within the United States.

Moreover, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or the USPTO, or become involved in opposition, derivation, reexamination, reissue, inter partes review, post grant review, interference proceedings or other patent office proceedings, court litigation or International Trade Commission proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation concerning our patent rights could reduce the scope of or prevent the enforceability of, or invalidate, our patent rights, allowing third parties to commercialize our technology or products, or equivalent or similar technology or products, and so to compete directly with us, without payment to us, or, where such proceedings involve third-party patents, result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened or narrowed by operation of any of the foregoing, such an event could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with adequate protection to prevent competitors from competing with us or otherwise to provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar, improved or alternative technologies or products in a non-infringing manner.

For example, although ridinilazole is protected by a U.S. composition of matter patent that recites hydrated forms of ridinilazole and a pharmaceutically acceptable excipient, and a method of treatment patent for *Clostridium difficile* associated disease, patent protection is not available for composition-of-matter claims that only recite the active pharmaceutical ingredient for ridinilazole without limitation to its use. Because ridinilazole lacks composition-of-matter protection for its active pharmaceutical ingredient, competitors will, subject to obtaining marketing approval, be able to offer and sell products with the same active pharmaceutical ingredient so long as these competitors do not infringe any other issued patents that would otherwise cover the drug's usage, methods

of treatment using the drug, drug formulations, drug dosage forms and the like. Moreover, method-of-treatment patent claims are more difficult to enforce than composition-of-matter claims for reasons including off-label sale, potential divided infringement issues and use of the subject compound in non-infringing manners. Physicians are permitted to prescribe an approved product for uses that are not described in the product's labeling. Although off-label prescriptions may infringe our method-of-treatment patents, the practice is common across medical specialties and such infringement is difficult to prevent or prosecute. Off-label sales would limit our ability to generate revenue from the sale of our product candidates, if approved for commercial sale. In addition, if a third party were able to design around our dosage-form and formulation patents and create a different formulation and dosage form that is not covered by our patents or patent applications, we would likely be unable to prevent that third party from manufacturing and marketing its product.

In addition, other companies may attempt to circumvent any regulatory data protection or market exclusivity, such as orphan drug exclusivity in the United States, which we obtain under applicable legislation, which may require us to allocate significant resources to preventing such circumvention. Legal and regulatory developments in the European Union and elsewhere may also result in clinical trial data submitted as part of a marketing authorization application becoming publicly available. Such developments could enable other companies to use our clinical trial data to assist in their own product development and to obtain marketing authorizations in the European Union and in other jurisdictions. Such developments may also require us to allocate significant resources to prevent other companies from circumventing or violating our intellectual property rights. Our attempts to prevent third parties from circumventing our intellectual property and other rights may ultimately be unsuccessful. We may also fail to take the required actions or pay the necessary fees to maintain our patents.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Future changes in U.S. statutory or case law beyond our control could affect some or all of the foregoing possibilities. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. This could be the case even after giving effect to patent term extensions and data exclusivity provisions preventing third parties from relying on clinical trial data filed by us for regulatory approval in support of their own applications for such approval. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

We may become involved in lawsuits or other enforcement proceedings to protect or enforce our patents or other intellectual property, which could be expensive, time-consuming and potentially unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file claims, which can be expensive and time-consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their intellectual property or that our patent and other intellectual property rights are invalid or unenforceable, including for anti-trust reasons. As a result, in a patent infringement proceeding, a court or administrative body may decide that a patent of ours is invalid or unenforceable, in whole or in part, or may construe the patent's claims narrowly and so refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the competitor technology in question. Even if we are successful in a patent infringement action, the unsuccessful party may subsequently raise antitrust issues and bring a follow-on action thereon. Antitrust issues may also provide a bar to settlement or constrain the permissible settlement terms. Further, settlement agreements in the pharmaceutical sector are the subject of ongoing review by the antitrust authorities in the European Union.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the intellectual property and other proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference, derivation, inter partes review, reexamination, reissue or post-grant review proceedings before the USPTO. The risks of being involved in such litigation and office proceedings may also increase as our product candidates approach commercialization, and as we gain greater visibility as a publicly traded company in the United States. Third parties may assert infringement claims against us based on existing or future intellectual property rights and so restrict our freedom to operate. Third parties may also seek injunctive relief against us, whereby they would attempt to prevent us from practicing our technologies altogether pending outcome of any litigation against us. We may not be aware of all such intellectual property rights potentially relating to our product candidates prior to their assertion against us. For example, we have not conducted an in-depth freedom-to-operate search or analysis for ezutromid or ridinilazole. Any freedom-to-operate search or analysis previously conducted may not have uncovered all relevant patents and pending patent applications, and there may be pending or future patent applications that, if issued, would block us from commercializing ezutromid or ridinilazole. Thus, we do not know with certainty whether ezutromid, ridinilazole or any other product candidate or our commercialization thereof, does not and will not infringe any third party's intellectual property.

If we are found to infringe a third party's intellectual property rights, or in order to avoid or settle litigation, we could be required to obtain a license to enable us to continue developing and marketing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies as are licensed to us, and could require us to make substantial payments. Absent a license, we could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties, or claims that we derived our inventions from another, could have a similar negative impact on our business.

We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary or otherwise confidential information or know-how of others in their work for us, we may be subject to claims that we or these employees have without authorization used or disclosed intellectual property, including trade secrets or other proprietary or confidential information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while we typically require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us and agreeing to cooperate and assist us with securing and defending our intellectual property, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation could cause us to spend substantial resources and could distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our ADSs and ordinary shares. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development, sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Accordingly, costs and lost management time, as well as uncertainties resulting from the initiation and continuation of patent litigation or other proceedings, could have a material adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary and confidential information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. However, we cannot guarantee that we have executed these agreements with each party that may have or have had access to our trade secrets or that the agreements we have executed will provide adequate protection. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary or confidential information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets, particularly unpatented know-how, were to be obtained or independently developed by a competitor, our competitive position would be harmed.

Risks Related to Regulatory Approval and Marketing of our Product Candidates

Even if we complete the necessary clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired.

Our product candidates, including ezutromid and ridinilazole, and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us or our collaborators from commercializing the

product candidate. We have not received approval to market ezutromid, ridinilazole or any of our other future product candidates from regulatory authorities in any jurisdiction.

We have only limited experience in filing and supporting the applications necessary to obtain marketing approvals for product candidates and expect to rely on third-party contract research organizations to assist us in this process. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Regulatory authorities may determine that ezutromid, ridinilazole or any of our other future product candidates are not effective or only moderately effective, or have undesirable or unintended side effects, toxicities, safety profiles or other characteristics that preclude us from obtaining marketing approval or that prevent or limit commercial use.

The process of obtaining marketing approvals is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable. If we experience delays in obtaining approval or if we fail to obtain approval of our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenues will be materially impaired.

Our failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed in these other jurisdictions, and any approval we are granted for our product candidates in the United States and Europe would not assure approval of our product candidates in other jurisdictions.

In order to market and sell ezutromid, ridinilazole and our other future product candidates in foreign jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements in those jurisdictions. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ from that required to obtain FDA or EMA approval. The regulatory approval process outside the United States and Europe generally includes all of the risks associated with obtaining FDA and EMA approval. In addition, some countries outside the United States and Europe require approval of the sales price of a drug before it can be marketed. In many countries, separate procedures must be followed to obtain reimbursement. We may not obtain marketing, pricing or reimbursement approvals outside the United States and Europe on a timely basis, if at all. Approval by the FDA or the EMA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States and Europe does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA or the EMA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. Marketing approvals in countries outside the United States and Europe do not ensure pricing approvals in those countries or in any other countries, and marketing approvals and pricing approvals do not ensure that reimbursement will be obtained.

Our ability to obtain and maintain conditional marketing authorizations in the European Union is limited to specific circumstances and subject to several conditions and obligations. A failure to renew any conditional approval that we obtain prior to full approval for the applicable indication would prevent us from continuing to market our products.

Conditional marketing authorizations based on incomplete clinical data may be granted for a limited number of listed medicinal products for human use, including products designated as orphan medicinal products under E.U.

law, if (1) the risk-benefit balance of the product is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) unmet medical needs will be fulfilled and (4) the benefit to public health of the immediate availability on the market of the medicinal product outweighs the risk inherent in the fact that additional data are still required. Specific obligations, including with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data, may be specified in the conditional marketing authorization. Conditional marketing authorizations are valid for one year and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions. Even if we, or a third-party collaborator, obtain conditional approval for ezutromid for the treatment of DMD or ridinilazole for the treatment of CDI, we or they may not be able to renew such conditional approval.

Even if we obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we manufacture and market our products and compliance with such requirements may involve substantial resources, which could materially impair our ability to generate revenue.

Even if marketing approval of a product candidate is granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation, including the requirement to implement a risk evaluation and mitigation strategy or to conduct costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. We and our collaborators must also comply with requirements concerning advertising and promotion for any of our product candidates for which we obtain marketing approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, neither we nor our collaborators will be able to promote any products we develop for indications or uses for which they are not approved. In addition, manufacturers of approved products and those manufacturers' facilities are required to ensure that quality control and manufacturing procedures conform to cGMP, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We and our contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMP.

Accordingly, assuming we receive marketing approval for one or more of our product candidates, we and our contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we are not able to comply with post-approval regulatory requirements, we could have the marketing approvals for our products withdrawn by regulatory authorities and our ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Thus, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any product candidate for which we obtain marketing approval will be subject to strict enforcement of post-marketing requirements and we could be subject to substantial penalties, including withdrawal of our product from the market, if we fail to comply with all regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include, but are not limited to, restrictions governing promotion of an approved product, submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping.

The FDA and other federal and state agencies, including the Department of Justice, closely regulate compliance with all requirements governing prescription drug products, including requirements pertaining to marketing and

promotion of drugs in accordance with the provisions of the approved labeling and manufacturing of products in accordance with cGMP requirements. The FDA and DOJ impose stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of such requirements may lead to investigations alleging violations of the Food, Drug and Cosmetic Act and other statutes, including the False Claims Act and other federal and state health care fraud and abuse laws as well as state consumer protection laws. Our failure to comply with all regulatory requirements, and later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, may yield various results, including:

- litigation involving patients taking our products;
- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance by us or any future collaborator with regulatory requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with regulatory requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Non-compliance with E.U. requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process.

If a drug is intended for the treatment of a serious or life threatening condition and the drug demonstrates the potential to address unmet medical need for this condition, the drug sponsor may apply for FDA fast track designation. The FDA has granted fast track designation for ezutromid and ridinilazole. However, a fast track designation does not ensure that either ezutromid or ridinilazole will receive marketing approval or that approval will be granted within any particular timeframe. We may also seek fast track designation for other future product

candidates. Even if the FDA grants fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of our product candidates.

If the FDA determines that a product candidate offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. Because the FDA designated ridinilazole as a qualified infectious disease product, or QIDP, ridinilazole also will receive priority review. We may also request priority review for ezutromid or other future product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

We may not be able to obtain orphan drug exclusivity for our product candidates. If our competitors are able to obtain orphan drug exclusivity for their products that are the same drug as our product candidates, or can be classified as a similar medicinal product within the meaning of E.U. law, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including Europe and the United States, may designate drugs for relatively small patient populations as orphan drugs. The FDA has granted orphan drug designation to ezutromid for the treatment of DMD, and the EMA has designated ezutromid as an orphan medicinal product. Generally, if a product with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of market exclusivity, which, subject to certain exceptions, precludes the EMA from accepting another marketing application for a similar medicinal product or the FDA from approving another marketing application for the same drug for the same indication for that time period. The applicable market exclusivity period is seven years in the United States and ten years in the European Union. The E.U. exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation, including if the drug is sufficiently profitable so that market exclusivity is no longer justified.

In the European Union, a "similar medicinal product" is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. For a drug such as ezutromid, the FDA defines "same drug" as a drug that contains the same active moiety and is intended for the same use. Obtaining orphan drug exclusivity for ezutromid for DMD, both in the United States and in Europe, may be important to the product candidate's success. If a competitor obtains orphan drug exclusivity for and approval of a product with the same indication as ezutromid before we do and if the competitor's product is the same drug or a similar medicinal product as ours, we could be excluded from the market.

Moreover, even if we obtain orphan drug exclusivity for ezutromid for DMD, we may not be able to maintain it. For example, if a competitive product that is the same drug or a similar medicinal product as our product candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product. In addition, orphan drug exclusivity will not prevent the approval of a product that is the same drug as our product candidate if the FDA finds that we cannot assure the availability of sufficient quantities of the drug to meet the needs of the persons with the disease or condition for which the drug was designated. Finally, even if we obtain orphan drug exclusivity for a product,

that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition.

Although the FDA has designated ezutromid for the treatment of DMD as a rare pediatric disease, that designation will not expedite approval of ezutromid nor will it ensure that we receive a Priority Review Voucher if ezutromid is approved by the FDA for the treatment of DMD.

Under the Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher, which can be redeemed to receive a priority review of a subsequent marketing application for a different product. The Priority Review Voucher is requested at the time of the marketing application and awarded upon approval of the product. The voucher may only be used once, but may be sold or transferred an unlimited number of times.

In September 2016, the FDA notified us that we obtained rare pediatric disease designation for ezutromid for the treatment of DMD. The FDA's rare pediatric disease designation gives us the potential to receive a Priority Review Voucher if ezutromid receives regulatory approval. Under the 21st Century Cures Act, the Rare Pediatric Disease Priority Review Voucher program was reauthorized until 2020. However, if a drug is designated before October 1, 2020, it is eligibile to receive a voucher if approved before October 2022. As a result, unless the program is futher extended, we will not receive a Priority Review Voucher if ezutromid is approved after October 2022. Moreover, even if we do receive a voucher, it may not be used to secure priority review of ezutromid for the treatment of DMD since it would only be issued upon approval of that product.

Our relationships with customers, healthcare providers and professionals and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any product candidates, including ezutromid or ridinilazole, for which we obtain marketing approval. Our future arrangements with customers, healthcare providers and professionals and third-party payors may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include, and are not limited to, the following:

- The federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federally funded healthcare programs such as Medicare and Medicaid. This statute has been broadly interpreted to apply to manufacturer arrangements with prescribers, purchasers and formulary managers, among others. Several other countries, including the United Kingdom, have enacted similar anti-kickback, fraud and abuse, and healthcare laws and regulations.
- The federal False Claims Act imposes civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. The government and qui tam relators have brought False Claims Act actions against pharmaceutical companies on the theory that their practices have caused false claims to be submitted to the government. There is also a separate false claims provision imposing criminal penalties.
- The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil

liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.

- HIPAA also imposes criminal liability for knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services.
- The federal Physician Sunshine Act requirements under the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, referred to together as the Affordable Care Act, require manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value made to or at the request of covered recipients, such as physicians and teaching hospitals, and physician ownership and investment interests in such manufacturers. Payments made to physicians and research institutions for clinical trials are included within the ambit of this law.
- Analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. Exclusion, suspension and debarment from government funded healthcare programs would significantly impact our ability to commercialize, sell or distribute any drug. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of ezutromid, ridinilazole or any of our other future product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates, including ezutromid or ridinilazole, for which we obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own

reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively the ACA. Among the provisions of the ACA of potential importance to our business are the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expansion of healthcare fraud and abuse laws, including the civil False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to individuals enrolled in Medicaid managed care organizations;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report certain financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- a new Independent Payment Advisory Board, or IPAB, which has authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs; and
- established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models.

Other legislative changes have been proposed and adopted since the ACA was enacted. These changes include the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year that started in 2013 and, due to subsequent legislation, will continue until 2025. In addition, the American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Moreover, the Medicare Access and CHIP Reauthorization Act of 2015, among other things, introduced the Quality Payment Program under which Medicare physicians will be required to either participate in an Advanced Alternative Payment Model, or AAPM, and assume some risk for patient outcomes, or participate in the Merit-Based Incentive Payment System, or MIPS, which will provide an incentive compensation structure that will rate physicians in part based on cost of services. These new laws may result in

additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

In addition, with the new Administration and Congress, there will likely be additional legislative changes, including repeal and replacement of certain provisions of the ACA. It remains to be seen, however, precisely what the new legislation will provide, when it will be enacted and what impact it will have on the availability of healthcare and containing or lowering the cost of healthcare. Such reforms could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop or commercialize product candidates. For example, the President and congressional leaders have expressed interest in repealing certain ACA provisions and replacing them with alternatives that may be less costly and provide state Medicaid programs and private health plans more flexibility. It is possible that these repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. The scope of potential future legislation to repeal and replace ACA provisions is highly uncertain in many respects, and it is possible that some of the ACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with ACA coverage expansion provisions.

Moreover, legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the United States Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and any collaborators to more stringent product labeling and post-marketing testing and other requirements.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our products. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, which could adversely affect our business, results of operations and financial condition.

Our operations are subject to anti-corruption laws, including the U.K. Bribery Act 2010, or Bribery Act, the U.S. Foreign Corrupt Practices Act, or FCPA, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The Bribery Act, FCPA and these other laws generally prohibit us, our officers, and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. We may in the future operate in jurisdictions that pose a high risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could

potentially subject us to liability under the Bribery Act, FCPA or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted.

We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United Kingdom and the United States, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, collectively referred to as the Trade Control laws.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the Bribery Act, the FCPA or other legal requirements, including Trade Control laws. If we are not in compliance with the Bribery Act, the FCPA and other anti-corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. Likewise, any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws or Trade Control laws by U.K., U.S. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

Risks Related to Employee Matters and Managing Growth

Our future success depends on our ability to retain our chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the principal members of our executive and scientific teams, including Glyn Edwards, our Chief Executive Officer, Erik Ostrowski, our Chief Financial Officer, Dr. David Roblin, our Chief Operating Officer and President of Research and Development, Dr. Ralf Rosskamp, our Chief Medical Officer, Dr. Jonathon Tinsley, our Chief Scientific Officer, DMD, and Dr. Richard Vickers, our Chief Scientific Officer, Antimicrobials. Although we have formal employment agreements with each of our executive officers, these agreements do not prevent our executives from terminating their employment with us at any time. We do not maintain "key person" insurance on any of our executive officers. The unplanned loss of the services of any of these persons could materially impact the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel, including in the United States where we plan to continue to expand our physical presence, will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous biotechnology and pharmaceutical companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

We expect to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train

additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Our employees may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA or Office of Inspector General regulations or similar regulations of comparable non-U.S. regulatory authorities, provide accurate information to the FDA or comparable non-U.S. regulatory authorities, comply with manufacturing standards we have established, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable non-U.S. regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

Risks Related to Ownership of our American Depositary Shares and Ordinary Shares

The prices of our ADSs and ordinary shares may be volatile and fluctuate substantially, which could result in substantial losses for holders of our ADSs and ordinary shares.

The market prices of our ADSs on the NASDAQ Global Market and of our ordinary shares on AIM may be volatile and fluctuate substantially. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, holders of our ADSs and ordinary shares may not be able to sell their ADSs or ordinary shares at or above the price at which they were purchased. The market price for the ADSs and ordinary shares may be influenced by many factors, including:

- the success of competitive products or technologies;
- results of clinical trials of ezutromid, ridinilazole and any other future product candidate that we develop;
- results of clinical trials of product candidates of our competitors;
- changes or developments in laws or regulations applicable to ezutromid and ridinilazole and any other future product candidates that we develop;
- our entry into, and the success of, any collaboration agreements with third parties;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;

- changes in the structure of healthcare payment systems;
- market conditions in the biotechnology and pharmaceutical sectors;
- general economic, industry and market conditions;
- the trading volume of ADSs on the NASDAQ Global Market and of our ordinary shares on AIM; and
- the other factors described in this "Risk Factors" section.

The dual listing of our ordinary shares and the ADSs may adversely affect the liquidity and value of the ADSs.

Our ADSs are traded on the NASDAQ Global Market, and our ordinary shares are listed on AIM. The dual listing of our ordinary shares and the ADSs may dilute the liquidity of these securities in one or both markets and may adversely affect the maintenance of an active trading market for the ADSs in the United States. The price of the ADSs could also be adversely affected by trading in our ordinary shares on AIM. Although our ordinary shares are currently listed on AIM, we may decide at some point in the future to delist our ordinary shares from AIM, and our ordinary shareholders may approve such delisting. We cannot predict the effect such delisting of our ordinary shares on AIM would have on the market price of the ADSs on the NASDAQ Global Market.

Securities traded on AIM may carry a higher risk than shares traded on other exchanges that may impact the value of your investment.

Our ordinary shares are currently traded on AIM. Investment in equities traded on AIM is perceived by some to carry a higher risk than an investment in equities quoted on exchanges with more stringent listing requirements, such as the London Stock Exchange, New York Stock Exchange or the NASDAQ Stock Market. This is because AIM imposes less stringent corporate governance and ongoing reporting requirements than those other exchanges. In addition, AIM requires only semi-annual, rather than quarterly, financial reporting. You should be aware that the value of our ordinary shares may be influenced by many factors, some of which may be specific to us and some of which may affect AIM-listed companies generally, including the depth and liquidity of the market, our performance, a large or small volume of trading in our ordinary shares, legislative changes and general economic, political or regulatory conditions, and that the prices may be volatile and subject to extensive fluctuations. Therefore, the market price of our ordinary shares underlying the ADSs may not reflect the underlying value of our company.

Substantial future sales of our ordinary shares or the ADSs in the public market, or the perception that these sales could occur, could cause the price of the ADSs to decline significantly, even if our business is doing well.

Sales of a substantial number of our ordinary shares or ADSs in the public market could occur at any time. These sales, or the perception in the market that these sales could occur, could cause the market price of our ADSs and ordinary shares to decline. The ordinary shares held by our major shareholders are available for sale and are not subject to contractual and legal restrictions on resale. If any of our directors, officers or major shareholders seek to sell substantial amounts of our ADSs or ordinary shares, particularly if these sales are in a rapid or disorderly manner, or other investors perceive that these sales could occur, the market price of our ADSs and ordinary shares could decrease significantly.

Holders of ADSs may not have the same voting rights as the holders of our ordinary shares and may not receive voting materials in time to be able to exercise their right to vote.

Except as provided in the deposit agreement relating to the ADSs, holders of the ADSs will not be able to exercise voting rights attaching to the ordinary shares evidenced by the ADSs. Holders of the ADSs will have the right to instruct the depositary with respect to the voting of the ordinary shares represented by the ADSs. If we tell the depositary to solicit your voting instructions, the depositary is required to endeavor to carry out your instructions. If we do not tell the depositary to solicit your voting instructions (and we are not required to do so),

you can still send instructions, and, in that case, the depositary may, but is not required to, carry out those instructions. You may not receive voting materials in time to instruct the depositary to vote, and it is possible that you, or persons who hold their ADSs through brokers, dealers or other third parties, will not have the opportunity to exercise a right to vote.

As a foreign private issuer, we are exempt from a number of rules under the U.S. securities laws and are permitted to file less information with the Securities and Exchange Commission than U.S. companies. This may limit the information available to holders of the ADSs.

We are a "foreign private issuer," as defined in the rules and regulations of the Securities and Exchange Commission, or the SEC, and, consequently, we are not subject to all of the disclosure requirements applicable to companies organized within the United States. For example, we are exempt from certain rules under the U.S. Securities Exchange Act of 1934, as amended, or the Exchange Act, that regulate disclosure obligations and procedural requirements related to the solicitation of proxies, consents or authorizations applicable to a security registered under the Exchange Act. In addition, our officers and directors are exempt from the reporting and "short-swing" profit recovery provisions of Section 16 of the Exchange Act and related rules with respect to their purchases and sales of our securities. Moreover, we are not required to file periodic reports and financial statements with the SEC as frequently or as promptly as U.S. public companies. Accordingly, there may be less publicly available information concerning our company than there is for public companies organized in the United States.

As a foreign private issuer, we will continue to file an annual report on Form 20-F within four months of the close of each fiscal year ending January 31 and reports on Form 6-K relating to certain material events promptly after we publicly announce these events. However, because of the above exemptions for foreign private issuers, our shareholders will not be afforded the same protections or information generally available to investors holding shares in public companies organized in the United States.

As a foreign private issuer, we are not subject to certain NASDAQ corporate governance rules applicable to public companies organized in the United States.

We rely on a provision in the NASDAQ Stock Market's Listed Company Manual that allows us to follow English company law in general and the U.K. Companies Act 2006 in particular with regard to certain aspects of corporate governance. This allows us to follow certain corporate governance practices that differ in significant respects from the corporate governance requirements applicable to U.S. companies listed on the NASDAQ Stock Market.

For example, we are exempt from regulations of the NASDAQ Stock Market that require listed companies organized in the United States to:

- have a majority of the board of directors consist of independent directors;
- require non-management directors to meet on a regular basis without management present;
- adopt a code of conduct and promptly disclose any waivers of the code for directors or executive officers that should address certain specified items;
- have an independent compensation committee;
- have an independent nominating committee;
- solicit proxies and provide proxy statements for all shareholder meetings;
- review related party transactions; and
- seek shareholder approval for the implementation of certain equity compensation plans and issuances of ordinary shares.

As a foreign private issuer, we are permitted to follow home country practice in lieu of the above requirements. Accordingly, our holders of ADSs and ordinary shares may not have the same protections afforded to shareholders of companies that are subject to these NASDAQ Stock Market requirements.

In accordance with our NASDAQ Stock Market listing, our Audit Committee is required to comply with the provisions of Section 301 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and Rule 10A-3 of the Exchange Act, both of which are also applicable to U.S. companies listed on the NASDAQ Stock Market. Because we are a foreign private issuer, however, our Audit Committee is not subject to additional requirements of the NASDAQ Stock Market applicable to listed U.S. companies, including an affirmative determination that all members of the Audit Committee are "independent," using more stringent criteria than those applicable to us as a foreign private issuer.

We may lose our foreign private issuer status in the future, which could result in significant additional costs and expenses.

As a "foreign private issuer" we are not required to comply with all the periodic disclosure and current reporting requirements of the Exchange Act and related rules and regulations. Under SEC rules, the determination of foreign private issuer status is made annually on the last business day of an issuer's most recently completed second fiscal quarter and, accordingly, the next determination will be made with respect to us on July 31, 2017.

In the future, we would lose our foreign private issuer status if a majority of our ordinary shares (including those represented by ADSs) are owned by U.S. shareholders and a majority of our shareholders, directors or management are U.S. citizens or residents and we fail to meet additional requirements necessary to avoid loss of foreign private issuer status. The regulatory and compliance costs to us under applicable U.S. securities laws as a U.S. domestic issuer may be significantly higher than our current regulatory and compliance costs. If we are not a foreign private issuer, we will be required to file periodic reports and registration statements on U.S. domestic issuer forms with the SEC, which are more detailed and extensive than the forms available to a foreign private issuer. For example, the annual report on Form 10-K requires domestic issuers to disclose executive compensation information on an individual basis with specific disclosure regarding the domestic compensation philosophy, objectives, annual total compensation (base salary, bonus, equity compensation) and potential payments in connection with change in control, retirement, death or disability, while the annual report on Form 20-F permits foreign private issuers to disclose compensation information on an aggregate basis. We will also have to report our results under U.S. Generally Accepted Accounting Principles, rather than under International Financial Reporting Standards, as a domestic registrant. We will also have to mandatorily comply with U.S. federal proxy requirements, and our officers, directors and principal shareholders will become subject to the short-swing profit disclosure and recovery provisions of Section 16 of the Exchange Act. We may also be required to modify certain of our policies to comply with corporate governance practices required for U.S. domestic issuers. Such conversion and modifications will involve additional costs. In addition, we may lose our ability to rely upon exemptions from certain corporate governance requirements of the NASDAQ Stock Market that are available to foreign private issuers.

We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make the ADSs less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company until January 31, 2021, or such earlier time that we are no longer an emerging growth company. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;

- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

We expect to continue to take advantage of some or all of the available exemptions. We cannot predict whether investors will find the ADSs less attractive if we rely on these exemptions. If some investors find the ADSs less attractive as a result, there may be a less active trading market for the ADSs and the market price of the ADSs may be more volatile.

In addition, the JOBS Act also provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We incur increased costs as a result of operating as a company with ADSs that are publicly traded in the United States, and our management is now required to devote substantial time to new compliance initiatives.

As a company with ADSs that are publicly traded in the United States, and particularly after we are no longer an "emerging growth company," we have incurred and will continue to incur significant legal, accounting and other expenses that we did not previously incur prior to our listing on the NASDAQ Stock Market. In addition, the Sarbanes-Oxley Act, the Dodd-Frank Act, the listing requirements of the NASDAQ Stock Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly.

However, for as long as we remain an emerging growth company, we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies as described in the preceding risk factor. We may remain an emerging growth company until January 31, 2021, although if the market value of our share capital held by non-affiliates exceeds $700 million as of any July 31 before that time or if we have annual gross revenues of $1 billion or more in any fiscal year, we would cease to be an emerging growth company as of January 31 of the applicable year. We also would cease to be an emerging growth company if we issue more than $1 billion of non-convertible debt over a three-year period.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, shareholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of the ADSs.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404 of the Sarbanes-Oxley Act, or Section 404, or any subsequent testing by our independent registered public accounting firm, as and when required, may reveal deficiencies in our internal controls over financial reporting

that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of the ADSs.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, as an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm until we are no longer an emerging growth company. To achieve compliance with Section 404 within the prescribed period, we are engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

We cannot assure you that we will not be classified as a passive foreign investment company for any taxable year, which may result in adverse U.S. federal income tax consequences to U.S. holders.

Based on our estimated gross income, the average value of our gross assets and the nature of our business, taking into account the market price of the ADSs, we do not believe that we were a "passive foreign investment company," or PFIC, for U.S. federal income tax purposes for our tax year ended January 31, 2017 and do not expect to be a PFIC during our tax year ending January 31, 2018. A corporation organized outside the United States generally will be classified as a PFIC for U.S. federal income tax purposes (1) in any taxable year in which at least 75% of its gross income is passive income or on average at least 50% of the gross value of its assets is attributable to assets that produce passive income or are held for the production of passive income and (2) as to a given holder who was a holder in such year and regardless of such corporation's income or asset composition in any subsequent taxable year unless, as to that holder, certain elections are made that can entail substantial tax costs to that holder. Passive income for this purpose generally includes dividends, interest, royalties, rents and gains from commodities and securities transactions. Our status in any taxable year will depend on our assets and activities in each year, and because this is a factual determination made annually after the end of each taxable year, there can be no assurance that we will not be considered a PFIC for the current taxable year or any future taxable year. The market value of our assets may be determined in large part by reference to the market price of the ADSs and our ordinary shares, which fluctuate and which may fluctuate considerably given that market prices of biotechnology companies have been especially volatile. If we were to be treated as a PFIC for any taxable year during which a U.S. holder held the ADSs, however, certain adverse U.S. federal income tax consequences could apply to the U.S. holder. See "Item 10.E Taxation."

U.S. investors may have difficulty enforcing civil liabilities against us, our directors or members of senior management and the experts named in this Annual Report.

Our directors and some of the experts named in this Annual Report are non-residents of the United States, and all or a substantial portion of the assets of such persons are located outside the United States. As a result, it may not be possible to serve process on such persons or us in the United States or to enforce judgments obtained in U.S. courts against them or us based on civil liability provisions of the securities laws of the United States. Further, there is doubt as to whether English courts would enforce certain civil liabilities under U.S. securities laws pursuant to judgments of U.S. courts based upon these civil liability provisions. In addition, awards of punitive damages in actions brought in the United States or elsewhere may be unenforceable in the United Kingdom. An award for monetary damages under the U.S. securities laws would be considered punitive if it does not seek to

compensate the claimant for loss or damage suffered and is intended to punish the defendant. The enforceability of any judgment in the United Kingdom will depend on the particular facts of the case as well as the laws and treaties in effect at the time. The United States and the United Kingdom do not currently have a treaty providing for recognition and enforcement of judgments (other than arbitration awards) in civil and commercial matters.

The rights of our shareholders may differ from the rights typically offered to shareholders of a U.S. corporation.

We are incorporated under U.K. law. The rights of holders of ordinary shares and, therefore, certain of the rights of holders of ADSs, are governed by U.K. law, including the provisions of the Companies Act 2006, and by our Articles of Association. These rights differ in certain respects from the rights of shareholders in typical U.S. corporations.

Holders of ordinary shares and ADSs may not receive a return on their ordinary shares or ADSs other than through the sale of their ordinary shares or ADSs.

Under current U.K. law, a company's accumulated realized profits must exceed its accumulated realized losses (on a non-consolidated basis) before dividends can be paid. Therefore, we must have distributable profits before issuing a dividend. We have not paid dividends in the past on our ordinary shares. We intend to retain earnings, if any, for use in our business and do not anticipate paying any cash dividends in the foreseeable future. Accordingly, other than through the sale of the ADSs or our ordinary shares, holders of such securities are unlikely to receive a return in the foreseeable future.

Holders of our ADSs may not receive distributions on our ordinary shares represented by the ADSs or any value for them if it is illegal or impractical to make them available to such holders.

The depositary for the ADSs has agreed to pay to holders of our ADSs or distribute the cash dividends or other distributions it or the custodian receives on our ordinary shares or other deposited securities after deducting its fees and expenses. Holders of our ADSs will receive these distributions in proportion to the number of our ordinary shares their ADSs represent. However, in accordance with the limitations set forth in the deposit agreement, it may be unlawful or impractical to make a distribution available to holders of our ADSs. We have no obligation to take any other action to permit the distribution of the ADSs, ordinary shares, rights or anything else to holders of the ADSs. This means that holders of our ADSs may not receive the distributions we make on our ordinary shares or any value from them if it is unlawful or impractical to make them available to such holders. These restrictions may have a material adverse effect on the value of our ADSs.

Our executive officers, directors and principal shareholders maintain the ability to control or significantly influence all matters submitted to stockholders for approval.

As of March 15, 2017, our executive officers, directors and principal shareholders beneficially owned, in the aggregate, ordinary shares and ADSs representing approximately 42.37% of our outstanding share capital. As a result, if these shareholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our shareholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control or significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other holders of ADSs and ordinary shares may desire.

In addition, and in accordance with the terms of our articles of association, our board maintains a classified board structure such that not all members of the board are elected at one time. All of our directors are subject to election by our shareholders at the first annual general meeting after their appointment to our board and to re-election by our shareholders at least once every three years thereafter. Because our board of directors is

responsible for appointing the members of our management team, this structure may frustrate or prevent any attempts by our shareholders to replace or remove our current management by making it more difficult for shareholders to replace members of our board of directors.

If equity research analysts stop publishing research or reports about our business or if they issue unfavorable commentary or downgrade the ADSs or our ordinary shares, the prices of the ADSs or our ordinary shares could decline.

The trading market for the ADSs and our ordinary shares relies in part on the research and reports that equity research analysts publish about us and our business. We do not control these analysts. The price of the ADSs or our ordinary shares could decline if one or more equity research analysts downgrades such securities or if analysts issue other unfavorable commentary about us or our business. In addition, if one or more of these analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause the trading prices and trading volumes of the ADS and our ordinary shares to decline.

We are exposed to risks related to currency exchange rates.

We conduct a significant portion of our operations outside of the United Kingdom. Because our financial statements are presented in pounds sterling, changes in currency exchange rates have had and could have a significant effect on our operating results when our operating results are translated into U.S. dollars. Exchange rate fluctuations between local currencies and the pound sterling create risk in several ways, including the following: weakening of the pound sterling may increase the pound sterling cost of overseas research and development expenses and the cost of sourced product components outside the United Kingdom; strengthening of the pound sterling may decrease the value of our revenues denominated in other currencies; the exchange rates on non-sterling transactions and cash deposits can distort our financial results; and commercial pricing and profit margins are affected by currency fluctuations.

We have broad discretion in the use of our cash and cash equivalents and may not use them effectively.

Our management has broad discretion in the use of our cash and cash equivalents and could spend our cash in ways that do not improve our results of operations or enhance the value of the ADSs and ordinary shares. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the market price of the ADSs and ordinary shares to decline and delay the development of our product candidates.

Item 4: Information on the Company

A. History and Development of the Company

We were founded in 2003 and are a public limited company incorporated under the laws of England and Wales with the Registrar of Companies of England and Wales, United Kingdom. Our principal office, which we moved to in March 2017, is located at 136a Eastern Avenue, Milton Park, Abingdon, Oxfordshire, OX14 4SB, and our telephone number is +(44) 1235 443 939. Our U.S. operations are conducted by our wholly owned subsidiary Summit Therapeutics Inc., a Delaware corporation. Our ordinary shares have traded on AIM, which is a sub-market of the London Stock Exchange, since October, 2004, under the symbol “SUMM” and our American Depositary Shares have traded on the NASDAQ Global Market since March 2015, under the symbol “SMMT.”

Our website address is www.summitplc.com. The information contained on, or that can be accessed from, our website does not form part of this Annual Report. Our agent for service of process in the United States is C T Corporation System, 111 Eighth Avenue, New York, New York 10011.

In the three-year period ended January 31, 2017, we have invested a total of £0.2 million in equipment and facilities.

B. Business

Overview

We are a biopharmaceutical company focused on the discovery, development and commercialization of novel medicines for indications for which there are no existing or only inadequate therapies. We are conducting clinical programs focused on the genetic disease Duchenne muscular dystrophy, or DMD, and the infectious disease *Clostridium difficile* infection, or CDI.

Duchenne Muscular Dystrophy

Our lead DMD product candidate is ezutromid (formerly SMT C1100), an orally administered small molecule. We are conducting a Phase 2 clinical trial of ezutromid in patients with DMD. This trial is designed to evaluate the potential benefits of longer-term dosing of ezutromid by measuring a number of endpoints related to muscle health and muscle function, along with monitoring the safety and tolerability of long-term exposure to ezutromid. We refer to this Phase 2 clinical trial as PhaseOut DMD, a Phase 2 proof of concept clinical trial. We expect to report 24-week biopsy data in the first quarter of 2018 for all patients in PhaseOut DMD who provide a 24-week biopsy sample. At the same time, we also expect to announce 24-week MRI and functional data analysis from all patients in the trial. Our DMD program is based on utrophin modulation, an approach to treating DMD that is independent of the underlying mutations in the dystrophin gene that cause the disease. We are a leader in the field of utrophin modulation, an approach that we believe has the potential to address the entire population of DMD patients. Other DMD approaches, such as exon-skipping and suppression of nonsense mutations, only address subsets of this population. The U.S. Food and Drug Administration, or the FDA, has granted orphan drug designation to ezutromid for the treatment of DMD, and the European Medicines Agency, or the EMA, has designated ezutromid as an orphan medicinal product. The FDA has also granted fast track designation and rare pediatric disease designation to ezutromid. In recent public statements, the FDA has stated that it recognizes the unmet medical need for DMD treatments, the devastating nature of the disease for patients and their families and the urgency to make new treatments available.

In October 2016, we entered into an exclusive license and collaboration agreement with Sarepta Therapeutics, Inc., or Sarepta, pursuant to which we granted Sarepta an exclusive license to commercialize our utrophin modulator pipeline, including ezutromid, in the European Union, Iceland, Norway, Switzerland, Turkey and the Commonwealth of Independent States, with an option to expand its commercial rights to include specified countries in Central and South America. We have retained commercialization rights to our utrophin modulator pipeline in the rest of the world.

DMD is one of the most common and the most severe form of muscular dystrophy. DMD predominantly affects males and results in the progressive wasting of muscles throughout the body. The disease typically results in death by the time DMD patients reach their late twenties. Individuals with DMD are unable to produce dystrophin, a protein essential for maintaining healthy muscle function.

Utrophin is a naturally occurring protein that is functionally and structurally similar to dystrophin. Utrophin plays an active role in the development of new muscle fibers, in particular during fetal development, and in repairing damaged muscle fibers. Utrophin production is down regulated, or switched off, in the late stages of gestation and can switch on and off as needed to repair damaged muscle. We believe that our approach of utrophin modulation can be used to maintain the production of utrophin in all skeletal muscles, including the diaphragm, and the heart to compensate for the lack of dystrophin in DMD patients, thereby restoring and maintaining healthy muscle function. This approach to treating DMD is independent of the underlying dystrophin gene mutation, and we believe has the potential to treat the entire population of DMD patients.

To date, we have conducted four Phase 1 clinical trials of ezutromid. We completed a Phase 1 clinical trial in healthy volunteers in 2012, a Phase 1b clinical trial in DMD patients in May 2014 and another Phase 1b clinical trial in DMD patients in September 2015. In addition, we completed a Phase 1 clinical trial that evaluated a new

formulation of ezutromid, which we refer to as the "F6" formulation, in healthy volunteers and DMD patients in August 2016. The second Phase 1b clinical trial of ezutromid in DMD patients evaluated the impact of diet on plasma levels of the drug. We refer to this second Phase 1b trial as our Phase 1b modified diet trial. In all of our Phase 1 trials, ezutromid was generally well tolerated at all doses tested.

We are also currently pursuing a broad utrophin modulator technology program to develop future generation utrophin modulator product candidates. This development is being undertaken as part of a strategic alliance with research groups at the University of Oxford.

Clostridium difficile Infection

Our lead CDI product candidate is ridinilazole (formerly SMT19969), an orally administered small molecule antibiotic. We reported positive top-line results from a Phase 2 clinical trial of ridinilazole in November 2015. Ridinilazole is designed to selectively target *Clostridium difficile* bacteria without causing collateral damage to the gut flora and thereby reduce CDI recurrence rates, which is the key clinical issue in this disease. The FDA has designated ridinilazole as a qualified infectious disease product, or QIDP, and the FDA granted ridinilazole fast track status. In 2013, the Centers for Disease Control and Prevention of the U.S. Department of Health and Human Services, or CDC, highlighted CDI as one of three pathogens that pose an immediate public health threat and require urgent and aggressive action.

CDI is a bacterial infection of the colon that produces toxins causing inflammation of the colon and severe diarrhea. CDI can also result in more serious disease complications, including pseudomembranous colitis, bowel perforation, toxic megacolon and sepsis. CDI typically develops following the use of broad spectrum antibiotics that can cause widespread damage to the natural gut flora and allow overgrowth of *Clostridium difficile* bacteria. CDI represents a serious healthcare issue in hospitals, long-term care homes and, increasingly, in the wider community. A study published in 2012 in *Clinical Infectious Diseases*, a peer reviewed journal published by the Infectious Diseases Society of America, estimated that CDI-related acute care costs total $4.8 billion per year in the United States alone.

We completed a Phase 1 clinical trial of ridinilazole in healthy volunteers in 2013. In this Phase 1 clinical trial, ridinilazole was highly selective for total clostridia bacteria with minimal impact on the other gut flora of subjects, which was consistent with the results of our preclinical studies of ridinilazole. In November 2015, we reported top-line results from our double blind, randomized, active controlled Phase 2 clinical trial that evaluated ridinilazole compared to the current standard of care, vancomycin, for the treatment of CDI. The Phase 2 clinical trial exceeded its primary endpoint of non-inferiority, with ridinilazole achieving statistical superiority over vancomycin in sustained clinical response, or SCR. The statistical superiority was driven by a large numerical reduction in recurrent disease compared with vancomycin. We subsequently reported that data from our Phase 2 clinical trial of ridinilazole showed ridinilazole to be highly preserving of the gut microbiome compared to patients who received vancomycin and experienced substantial damage to their gut microbiome that for many patients persisted during the 30-day post-treatment period. Ridinilazole was well tolerated at all doses tested in both Phase 1 and Phase 2 clinical trials. We are also conducting an exploratory open label, active controlled Phase 2 clinical trial evaluating ridinilazole compared to fidaxomicin. We have completed treating patients in this trial and expect to report top-line results in the second quarter of 2017.

In February 2017, following regulatory meetings with the FDA and EMA, we outlined our planned Phase 3 clinical development program for ridinilazole. We expect the program will consist of two Phase 3 clinical trials comparing ridinilazole to vancomycin with the primary endpoint in both trials being superiority in SCR. We plan to commence the Phase 3 clinical trials in the first half of 2018. We are currently exploring funding options for the Phase 3 clinical development program for ridinilazole, including third party collaboration or non-dilutive government or charitable organization funding. We hold exclusive worldwide commercialization rights for ridinilazole.

Our Product Development Pipeline

The following table summarizes our product development pipeline. We are also developing an earlier stage pipeline of future generation utrophin modulators for the treatment of DMD.

Program	Preclinical	Phase 1	Phase 2	Phase 3	Expected Results
***Clostridium difficile* Infection**					
Ridinilazole (formerly SMT19969)	*Qualified Infectious Disease Product Designation (FDA)* *Fast Track Designation (FDA)*				Expect to report top-line results from Phase 2 clinical trial evaluating ridinilazole compared to fidaxomicin in the second quarter of 2017.
Duchenne Muscular Dystrophy					
Ezutromid† (formerly SMT C1100)	*Orphan Drug Designation (FDA)* *Orphan Medicinal Product Designation (EMA)* *Fast Track Designation (FDA)* *Rare Pediatric Disease Designation (FDA)*				Expect to report 24-week biopsy data in the first quarter of 2018 for all patients who provide a 24-week biopsy sample.

† We have granted Sarepta an exclusive license to the commercial rights for our utrophin modulator pipeline, including ezutromid, in the European Union, Iceland, Norway, Switzerland, Turkey and the Commonwealth of Independent States, with an option to expand its commercial rights to include specified countries in Central and South America. We retain commercialization rights in the rest of the world.

Our Strategy

Our goal is to become a fully integrated biopharmaceutical company focused on the discovery, development and commercialization of novel medicines for indications for which there are no existing or only inadequate therapies, with a current focus on DMD and CDI. The key elements of our strategy to achieve this goal are:

Rapidly advance the development of our lead product candidates, ezutromid for DMD and ridinilazole for CDI.

We are focusing our resources and business efforts primarily on rapidly advancing the development of ezutromid for the treatment of DMD and ridinilazole for the treatment of CDI. We believe that there is significant market potential for each of these product candidates. We also believe that the orphan drug and fast track designations of ezutromid and the QIDP and fast track designations of ridinilazole may expedite the regulatory review process for each of these product candidates and potentially provide market protection benefits. We are currently conducting our PhaseOut DMD clinical trial. This Phase 2 clinical trial is evaluating the benefits of longer-term dosing of ezutromid by measuring a number of endpoints related to muscle health and muscle function, including distribution of utrophin protein in muscle fibers and levels of muscle fiber regeneration from muscle biopsies, changes in muscle inflammation and fat infiltration through the use of magnetic resonance imaging, or MRI, the distance walked during the six minute walk test and the North Star Ambulatory Assessment, a multi-point test of motor functions. We expect to report 24-week biopsy data in the first quarter of 2018 for all patients in PhaseOut DMD who provide a 24-week biopsy sample. At the same time, we also expect to announce 24-week MRI and functional data analysis from all patients in the trial. We reported top-line data from our double blind, randomized, active controlled Phase 2 clinical trial that evaluated ridinilazole compared to vancomycin for the

treatment of CDI in November 2015. The primary endpoint of the trial was exceeded with ridinilazole achieving statistical superiority over vancomycin in sustained clinical response, with this superiority being driven by a large numerical reduction in recurrent disease compared to vancomycin. We are undertaking activities required to advance ridinilazole into Phase 3 clinical trials and, as detailed below, are evaluating our options to maximize the commercial potential of ridinilazole.

Maintain and expand our leadership in the field of utrophin modulation.

We are developing ezutromid as the first of a new class of drugs called utrophin modulators. Utrophin modulation is an approach to treating DMD that is independent of the underlying dystrophin gene mutation. Our co-founder and scientific advisor, Professor Kay Davies at the University of Oxford, discovered utrophin and then developed the concept of utilizing utrophin modulation as a treatment potentially applicable to all DMD patients. Our DMD program was founded to develop and commercialize drugs for DMD using this approach to treatment. We plan to apply and enhance our existing knowledge, experience and proprietary rights to maintain and expand our leadership in the field of utrophin modulation. In addition to the PhaseOut DMD clinical trial for ezutromid, we are also currently pursuing, in collaboration with the University of Oxford, a broad utrophin modulator technology program consisting of novel, future generation, small molecule utrophin modulators with potential new utrophin-related mechanisms.

Collaborate with Sarepta on the global research, development and commercialization of our utrophin modulator pipeline.

We entered into an exclusive license and collaboration agreement with Sarepta in October 2016 pursuant to which we granted Sarepta the exclusive right to commercialize products in our utrophin modulator pipeline in the European Union, Iceland, Norway, Switzerland, Turkey and the Commonwealth of Independent States, which we refer to as the licensed territory. Such products include ezutromid and our future generation of small molecule utrophin modulators, which we refer to collectively as the licensed products. We have agreed to collaborate with Sarepta on the research and development of the licensed products under a joint, global development plan through a joint steering committee. Sarepta has the final decision making authority with respect to commercialization decisions of the licensed products in the licensed territories. We are working with Sarepta to implement the global development plan and fulfill our respective contractual obligations under the terms of the license and collaboration agreement that includes research and development activities, sharing of global research and development costs beginning in 2018, manufacture and supply of licensed product material, intellectual property, and commercialization activities.

Commercialize ezutromid for DMD in the United States with our own specialty commercial team.

We hold exclusive commercialization rights for ezutromid for all indications in the United States. If ezutromid receives marketing approval, we intend to commercialize it initially in the United States with our own focused, specialized sales force that we plan to establish. We believe that medical specialists treating DMD are sufficiently concentrated that we will be able to effectively promote ezutromid with a targeted sales team in the United States and potentially other territories. We also believe that our relationships with patient advocacy groups will strengthen our ability to market ezutromid. We also plan to evaluate the potential for utilizing collaboration, distribution and other marketing arrangements with third parties to commercialize ezutromid in the jurisdictions where we retain commercial rights.

Maximize the commercial potential of ridinilazole.

We hold exclusive worldwide commercialization rights for ridinilazole for all indications. We are evaluating our options to maximize the commercial opportunity for ridinilazole. We may seek third-party collaborators for the development and commercialization of ridinilazole or potentially retain commercialization rights for ourselves. We are also exploring funding options from government entities and philanthropic, non-government and not for profit organizations. In this evaluation, we are considering factors such as the anticipated development costs required to achieve marketing approval, the sales and marketing resources required in each territory in which we

receive approval, the relative size of the market opportunity in such territory, the particular expertise of the third party and the proposed financial terms of any arrangement with third party collaborators, government entities or philanthropic, non-government or not for profit organizations.

Seek additional governmental and other third party grants and support.

We have obtained development funding and other assistance from government entities, philanthropic, non-government and not for profit organizations and patient advocacy groups for our product candidates. For example, we have received grant funding and clinical trial support from Innovate UK and several DMD organizations, including groups based in the United States, such as the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, Charley's Fund, Cure Duchenne, Foundation to Eradicate Duchenne and the Nash Avery Foundation, and groups based in the United Kingdom, such as Joining Jack. The Wellcome Trust Limited provided funding for ridinilazole up until the completion of our Phase 2 proof of concept clinical trial. We plan to continue to encourage these types of organizations to provide additional funding and support for our development programs.

Duchenne Muscular Dystrophy Overview

Duchenne muscular dystrophy is one of the most common and the most severe form of muscular dystrophy. DMD is a fatal disease that results in progressive wasting of muscles throughout the body. DMD is caused by different genetic mutations affecting the dystrophin gene on the X-chromosome, and therefore predominately affects males. As a result of these genetic mutations, DMD patients are unable to produce dystrophin, a protein essential for maintaining healthy muscle function. Over time, the muscles of DMD patients deteriorate and are infiltrated by fat and scar tissue, which is referred to as fibrosis, leading to the loss of ambulation, loss of respiratory and cardiac function and ultimately death.

Based on prevalence data published in November 2016 by Orphanet, a publicly available reference portal for information on rare diseases and orphan drugs, we estimate that there are approximately 50,000 DMD patients in the developed world and 250,000 DMD patients globally. According to an article published in 2013 in the peer reviewed journal *Muscle & Nerve*, approximately one in every 5,000 males is born with DMD. All ethnic groups are generally susceptible to DMD at approximately the same rates. Approximately two-thirds of DMD cases are due to inherited mutations, with the remainder being the result of spontaneous mutations in the dystrophin gene in patients with no familial history of the disease.

DMD is typically diagnosed in patients who are between two and seven years of age. The onset of the physical symptoms can be difficult to recognize, but early indicators of disease due to muscle weakness include difficulty walking or jumping, frequent falling over and becoming fatigued more easily. A preliminary diagnosis is typically made by measuring blood plasma levels of the enzyme creatine kinase, or CK. CK levels in DMD children are often ten to 100 times higher than CK levels in non-DMD children. A diagnosis of DMD is then confirmed through genetic testing using blood cells or muscle biopsy. In the United States and Europe, there are a number of newborn screening studies that can diagnose DMD at birth, although these tests are not yet routinely performed.

Initially, DMD affects the skeletal muscles in the arms, legs and trunk. By around 12 years of age, most DMD patients will need to use a wheelchair on a regular basis. Significant loss of skeletal muscle function takes place during the teenage years, and, while greater assistance is needed for activities involving arms, legs or trunk, most patients will retain use of their fingers, allowing them to write or use computers. Symptoms of scoliosis, or curvature of the spine, may also develop due to loss of trunk muscle function.

In the later stages of disease progression, life threatening heart and respiratory conditions become common. The function of the diaphragm and muscles responsible for the mechanical aspects of breathing deteriorates, leading to shortness of breath and build-up of fluid in the lungs and requiring ventilation at night and eventually on a

24-hour basis. DMD patients also develop cardiomyopathy, or enlarged hearts. The failure of the cardiac and respiratory systems typically leads to death by the time DMD patients reach their late twenties.

Current DMD Treatments and Development Approaches

There is currently no approved therapy for the treatment of DMD applicable to all DMD patients that seeks to alter the progression of the disease. Corticosteroids are prescribed to DMD patients from a young age to help treat symptoms of the disease. However, long-term use of corticosteroids is associated with severe side effects and concerns over weight gain. Other treatments to manage the symptoms of the disease include regular physiotherapy, surgery and mechanical support, such as wheelchairs and leg braces, and dietary supplements.

There are different approaches in development for the treatment of DMD, some of which seek to alter the progression of the disease by targeting the underlying genetic cause and others that seek to provide symptomatic relief. One disease modifying treatment for DMD is based on a scientific approach known as exon-skipping. Exons are organic molecules known as nucleotides within the DNA strand that the cellular machinery translates to make truncated but functional protein. In a sub-population of DMD patients, synthesis of the dystrophin protein is disrupted because of mutations that may be due, among other factors, to deleted exons. Exon-skipping technology seeks to allow the production of a truncated but still functional dystrophin protein. According to an article published in 2009 in the peer reviewed journal *Human Mutation*, skipping of the ten most common exons would treat in aggregate approximately 41% of all DMD patients. There is currently one approved exon-skipping treatment in the United States, called eteplirsen (Exondys 51™), which is being developed and commercialized by Sarepta. Eteplirsen received accelerated approval from the FDA in September 2016, and based on the size of the DMD population described in the aforementioned article in *Human Mutation*, it has the potential to treat approximately 13% of patients with DMD. We believe that there are additional exon-skipping therapies currently in clinical development to address three additional exons and that these, in aggregate with eteplirsen, would treat less than one-third of all DMD patients. Another approach that seeks to alter the progression of the disease involves targeting the specific genetic mutations known as nonsense mutations. Nonsense mutations create a premature stop signal in the translation of the genetic code and prevent the production of functional dystrophin protein. There is currently one approved nonsense mutation treatment in Europe, called ataluren (Translarna™), which is being developed and commercialized by PTC Therapeutics Inc. Ataluren received conditional approval from the EMA in May 2014. DMD caused by nonsense mutations affects approximately 13% of all DMD patients. One other potential disease modifying treatment approach in development is gene therapy, which has the potential to address the genetic cause of DMD by using an adeno-associated virus to deliver a shortened, yet functional, version of the dystrophin gene to a DMD patient. A number of other treatments being developed seek to alleviate the symptoms of DMD. These include promotion of muscle tissue growth based on myostatin inhibition, anti-inflammatory and anti-fibrotic drugs and treatments to improve cardiac and respiratory function.

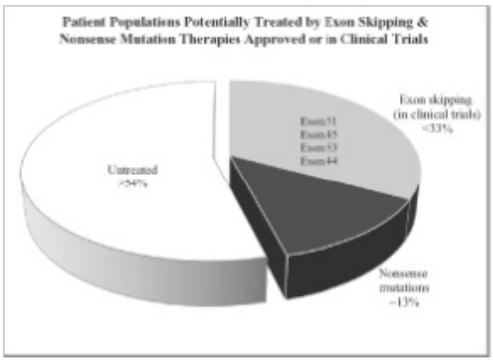
The FDA recognizes the unmet medical need in DMD, the devastating nature of the disease for patients and their families and the urgency to make new treatments available. The FDA publicly stated in October 2014 that it remains committed to working with all companies to expedite the development and approval of safe and effective drugs to treat this disease. The Director of the FDA's Center for Drug Evaluation and Research also stated in a speech in July 2014 that the agency was willing to explore the use of all potential pathways for approval of DMD drugs, including accelerated approval, as appropriate. In June 2015, the FDA issued draft guidance on developing drugs for the treatment of DMD and related dystrophinopathies.

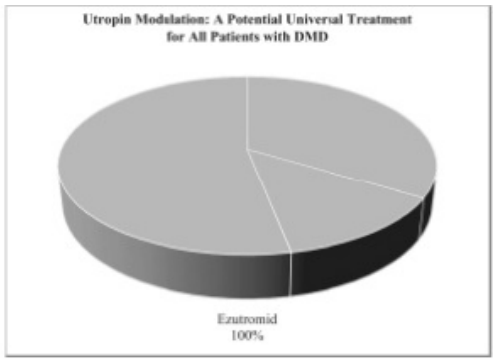
Our Utrophin Modulation Approach for the Treatment of DMD

Our Approach

We believe that our approach of utilizing utrophin modulation for DMD has the potential to slow or stop the progression of DMD in all patients with the disease. Utrophin is a naturally occurring protein that is functionally and structurally similar to dystrophin. The aim of utrophin modulation is to maintain the production of utrophin

in all skeletal muscles, including the diaphragm, and the heart to compensate for the lack of dystrophin in DMD patients, thereby restoring and maintaining healthy muscle function. This approach to treating DMD is independent of the underlying dystrophin gene mutation. As illustrated in the figure below, we believe utrophin modulation has the potential to treat the entire population of DMD patients, unlike other DMD approaches that also seek to alter the progression of the disease but only address subsets of the total DMD population.





Further, we believe utrophin modulation could potentially be complementary to potential treatments for DMD based on other scientific approaches, including approaches that are focused on restoring dystrophin, such as exon-skipping and suppression of nonsense mutations. We also expect that utrophin modulation has the potential to benefit patients with Becker muscular dystrophy, a milder form of the disease in which the majority of patients produce low levels of shortened dystrophin.

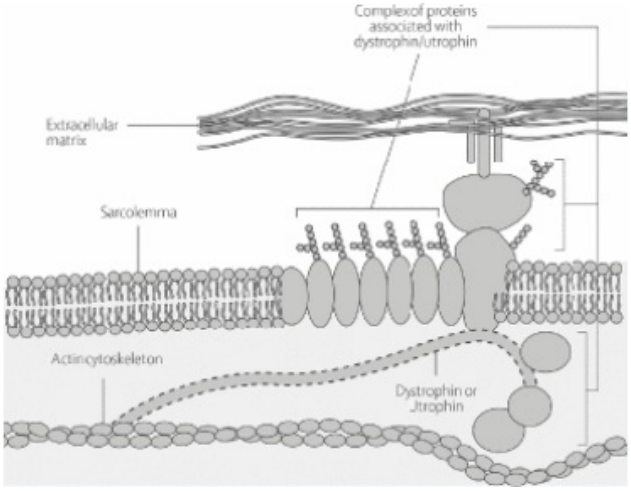
The Role of Utrophin and Dystrophin in Muscle Fibers

Utrophin and dystrophin are structurally and functionally similar proteins that perform a critical role in maintaining the proper function of muscle fibers, although at different times and in different settings. The roles of utrophin and dystrophin depend on whether the muscle fibers are mature, in the development stage or in the process of being repaired and regenerated. As discussed below, dystrophin plays an active role in maintaining the function of mature muscle fibers, while utrophin plays an active role in the development of new muscle fibers and in repairing damaged muscle fibers.

Role of Dystrophin in Mature Muscle

Each muscle in the body is made up of bundles of thousands of muscle fibers. Dystrophin and a group of different proteins that bind to dystrophin, which are called the Dystrophin Associated Protein Complex, or DAPC, are located at specific sites along the entire length of the muscle cell membrane, referred to as the sarcolemma, of every muscle fiber. Dystrophin works by linking the actin cytoskeleton, which is a part of the muscle fiber's contractile apparatus, to the DAPC in the sarcolemma. The DAPC, in turn, links the sarcolemma to the extracellular matrix, which binds the bundles of muscle fibers together. This link serves as a molecular shock absorber that helps to maintain stability and elasticity of muscle fibers during contraction and relaxation. In the absence of dystrophin, this linkage is lost and muscles become damaged, which leads to continual destructive rounds of muscle degeneration and regeneration and ultimately to progressive muscle wasting. The figure below depicts the DAPC and illustrates the role of dystrophin (or utrophin) and the other proteins that make up this complex.

The Role of Dystrophin or Utrophin in the Associated Protein Complex



Role of Utrophin in Developing Muscle

In both DMD patients and healthy individuals, utrophin and the proteins that comprise the DAPC are highly localized at specific sites along the length of muscle fibers during fetal development. Utrophin production is then down regulated, or switched off, in the late stages of gestation. In the normal muscle fiber of healthy individuals, the production of dystrophin begins to replace utrophin at these sites in the maturing muscle fiber, eventually fully replacing utrophin. In the muscle fiber of DMD patients, who are unable to produce functional dystrophin to substitute for the down regulating utrophin, these sites in the muscle fiber become unoccupied, which leads to muscle degeneration as muscles mature.

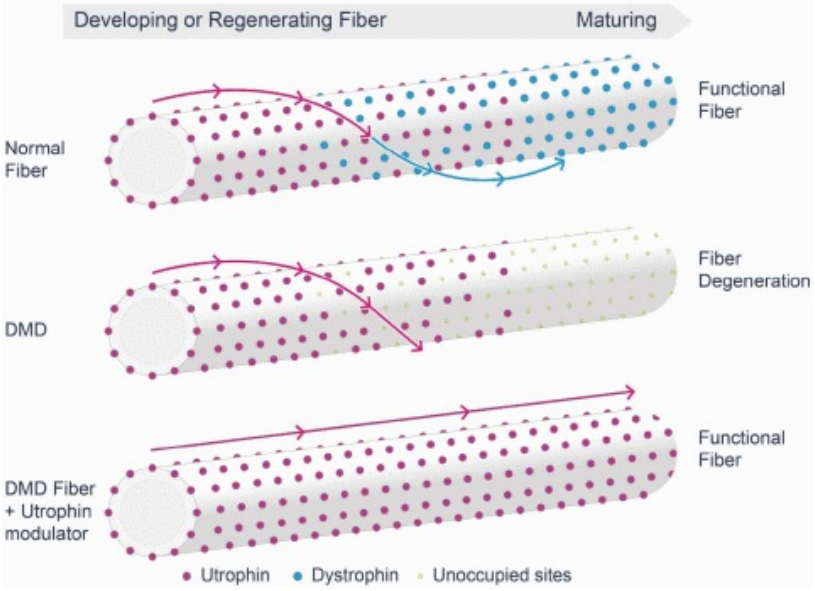
Role of Utrophin in Regenerating Muscle

In both DMD patients and healthy individuals, utrophin is localized to the neuromuscular junctions, which connect nerve fibers and muscles, and myotendinous junctions, which connect tendons and muscles. The other major role of utrophin in muscle fibers is to stabilize newly regenerating muscle fibers as part of the natural repair process. After a muscle fiber is damaged, utrophin production switches on as needed to repair the damaged region and then switches off following successful repair.

Expected Effect of Utrophin Modulation for DMD

We believe that our approach of utrophin modulation can be used to maintain the production of utrophin in maturing and mature muscle fibers and compensate for the lack of dystrophin in DMD patients, thereby restoring and maintaining healthy muscle function. The figure below illustrates the transition from utrophin to dystrophin production in the normal muscle fiber of a healthy individual, the effect of the lack of dystrophin production in

the muscle fiber of a DMD patient and the expected effect of utrophin modulation in the muscle fiber of a DMD patient to compensate for the lack of dystrophin production.



Origins of Our Utrophin Modulation Approach

Our co-founder and scientific advisor, Professor Kay Davies at the University of Oxford, discovered utrophin and then developed the concept of utilizing utrophin as a treatment potentially applicable to all DMD patients. Our DMD program was founded to develop and commercialize drugs for DMD using this approach to treatment. Professor Davies' research group at the University of Oxford developed transgenic lines of an *mdx* mouse that were genetically engineered to continually express utrophin protein. The *mdx* mouse is a naturally occurring animal model that is dystrophin deficient and is the standard disease model for studies of DMD. In these experiments, the continued expression of utrophin, even at levels just above those in a normal *mdx* mouse, had a meaningful, positive effect on muscle performance.

Our utrophin modulation program uses small molecule drugs that are designed to achieve the same effect seen in the transgenic *mdx* mouse experiments and to continually express utrophin to protect muscle fibers against DMD.

Ezutromid Overview

Our most advanced utrophin modulator product candidate is ezutromid, an orally administered small molecule.

To date, we have conducted four Phase 1 clinical trials of ezutromid. We completed a Phase 1 clinical trial of ezutromid in healthy volunteers in 2012, a Phase 1b clinical trial of ezutromid in DMD patients in May 2014 and another Phase 1b clinical trial of ezutromid in DMD patients in September 2015. In addition, we completed a Phase 1 clinical trial evaluating a new formulation of ezutromid, which we refer to as the "F6" formulation, in healthy volunteers and DMD patients in August 2016. The second Phase 1b clinical trial of ezutromid in DMD patients evaluated the current clinical formulation of ezutromid, which we refer to as the "F3" formulation, and the impact of diet on plasma levels of the drug. We refer to this second Phase 1b trial as our Phase 1b modified diet trial. Our Phase 1b modified diet trial met its primary objective with patients achieving plasma levels of

ezutromid that may be sufficient to modulate the production of utrophin protein and possibly result in clinical benefit while following specific dietary guidance. In our Phase 1 clinical trial evaluating the F6 formulation of ezutromid, the evaluable patients who received the highest dose achieved an over six-fold increase in the average maximum plasma concentration level compared to the F3 formulation of ezutromid.

In all four Phase 1 clinical trials, ezutromid was generally well tolerated at all doses tested. One patient in the Phase 1 clinical trial of the F6 formulation of ezutromid exhibited changes in liver parameters and withdrew from the trial, despite showing no clinical symptoms. The findings were classified as a serious adverse event. No other serious adverse events reported in our Phase 1 clinical trials of ezutromid.

We are conducting a Phase 2 clinical trial of ezutromid, which we refer to as PhaseOut DMD, in patients with DMD at trial sites in the United Kingdom and the United States. The PhaseOut DMD trial is evaluating the benefits of longer-term dosing of the F3 and F6 formulations of ezutromid by measuring a number of endpoints related to muscle health and muscle function, including distribution of utrophin protein in muscle fibers and levels of muscle fiber regeneration from muscle biopsies, changes in muscle inflammation and fat infiltration through the use of MRI, distance walked during a six minute walk test and the North Star Ambulatory Assessment. Based on our current estimates, we expect to complete enrollment for PhaseOut DMD in the second quarter of 2017, and we expect to report 24-week biopsy data in the first quarter of 2018 for all patients in PhaseOut DMD who provide a 24-week biopsy sample. At the same time, we also expect to announce 24-week MRI and functional data analysis from all patients in the trial.

We believe that the F3 and F6 formulations will be appropriate for administration to DMD patients, especially children.

The FDA has granted orphan drug designation to ezutromid for the treatment of DMD, and the EMA has designated ezutromid as an orphan medicinal product. In the United States, if a product with orphan designation receives FDA approval, the FDA will not approve a later product for the same indication that uses the same active ingredient for seven years, unless the later product is shown to be clinically superior. In the European Union, if an orphan medicinal product receives EMA approval, the EMA will not approve a later product for the same therapeutic indication and with the same method of action for ten years after the orphan medicinal product receives EMA approval, subject to certain exceptions, including if the later product demonstrates clinical superiority. The FDA has also granted ezutromid fast track designation and rare pediatric disease designation. Fast track designation provides companies with advantages such as opportunities for more frequent interactions with the FDA during all aspects of development, submission of a new drug application, or NDA, on a rolling basis, and eligibility for accelerated approval and priority review. With rare pediatric disease designation we could qualify for a priority review voucher upon the approval of ezutromid, which could be used for a subsequent marketing application or sold or transferred an unlimited number of times (although only used once).

Collaboration with Sarepta

In October 2016, we entered into an exclusive license and collaboration agreement with Sarepta. Under the terms of the agreement, we granted Sarepta the exclusive right to commercialize products in our utrophin modulator pipeline in the European Union, Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States, which we refer to as the licensed territory. Such products include ezutromid and our pipeline of future generation small molecule utrophin modulators, which we refer to as the licensed products. We also granted Sarepta an option to expand the licensed territory to include specified countries in Central and South America. We retained commercialization rights in the rest of the world.

Under the terms of the license and collaboration agreement, we received an upfront payment of $40.0 million from Sarepta. In addition, we will be eligible to receive future ezutromid-related development, regulatory and sales milestone payments totaling up to $522.0 million. This includes a $22.0 million milestone, payable on or after April 1, 2017, following the first dosing of the last patient in our PhaseOut DMD clinical trial. We will also

be eligible to receive development and sales milestone payments related to potential second generation and future generation utrophin modulator candidate(s). We are also eligible to receive escalating royalties ranging from a low to high teens percentage of net sales on a product-by-product basis in the licensed territories. If Sarepta elects to exercise its option for rights in specified countries in Central and South America, we would be entitled to additional fees, milestones and royalties.

We have agreed to collaborate with Sarepta on the research and development of licensed products under a joint, global development plan. Under the license and collaboration agreement, we will be solely responsible for all research and development costs for the licensed products until December 31, 2017. Thereafter, we will be responsible for 55.0% of all global budgeted research and development costs related to the licensed products, and Sarepta will be responsible for 45.0% of such costs. Sarepta has final decision making authority with respect to commercialization decisions of the licensed products in the licensed territories. Sarepta will be solely responsible for all commercialization activities and associated costs, relating to licensed products in the licensed territories.

Ezutromid Clinical Development

To date we have completed four Phase 1 clinical trials of ezutromid, which are summarized in the table below. The design and results of each clinical trial are discussed in more detail further below.

Trial	Description	Duration of Treatment	Total No. of Patients	No. of Patients Treated with Ezutromid
Phase 1 healthy volunteer trial (Trial 01)	Double blind, placebo controlled, ascending single and multiple oral dose trial	10 days	49	36
Phase 1b DMD patient trial (Trial 02)	Open label, ascending single and multiple oral dose trial	10 days	12	12
Phase 1b modified diet trial (Trial 03)	Double blind, randomized, placebo controlled multiple oral dose trial with dietary guidance	14 days	12	12
Phase 1 healthy volunteer and DMD patient trial (Trial 04)	Open label, ascending multiple oral dose trial with dietary guidance evaluating new F6 formulation of ezutromid	5 days (healthy volunteers) and 7 days (DMD patients)	24 (16 healthy volunteers, 8 DMD patients)	24 (16 healthy volunteers, 8 DMD patients)

Phase 1 Clinical Trial in Healthy Volunteers (Trial 01)

In 2012, we completed a double blind, placebo controlled, ascending single and multiple oral dose Phase 1 clinical trial of ezutromid in healthy volunteers. We conducted this clinical trial at a single site in the United Kingdom under approval from the Medicines and Healthcare products Regulatory Agency, or MHRA, and the U.K. Health Research Authority Ethics Review Committee, or the Ethics Review Committee. We enrolled 49 healthy male subjects who were between 18 and 55 years of age. Forty-seven subjects completed the clinical trial. Two subjects withdrew from the clinical trial for reasons unrelated to ezutromid.

The primary objective of the clinical trial was to determine the safety and tolerability of single and multiple oral doses of ezutromid in healthy male subjects. The secondary objectives were to determine the single and multiple oral dose pharmacokinetics of ezutromid based on the concentration of the drug in blood plasma and the effect of fasting on the single oral dose pharmacokinetics of ezutromid.

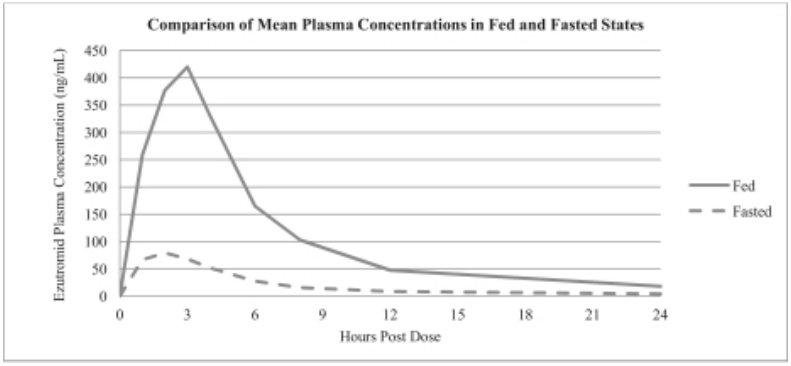
We conducted the clinical trial in two parts. Part 1 consisted of an ascending single dose study with a fasted effect evaluation. We evaluated a total of 32 subjects, who were divided into four equal cohorts of eight subjects each. Subjects in the four cohorts received one of the following doses of ezutromid: 50 mg/kg, 100 mg/kg, 200 mg/kg or 400 mg/kg. Six subjects in each cohort received ezutromid at the specified dose, and two subjects in each cohort received placebo. Each subject in the cohort receiving ezutromid at a dose of 200 mg/kg received doses under both fasted and fed conditions, while the subjects in the other cohorts received doses under normal, fed conditions, with no special dietary rules. One subject was removed during Part 1 of the clinical trial prior to dosing in a fasted state after testing positive for drug use.

Part 2 of the clinical trial consisted of a multiple ascending dose study. We evaluated a total of 16 subjects, who were divided into two cohorts of eight subjects each. In the first cohort, six subjects received 100 mg/kg doses of ezutromid and two subjects received placebo, in each case administered twice per day for ten days. In the second cohort, six subjects received 200 mg/kg doses of ezutromid and two subjects received placebo, in each case administered twice per day for ten days.

Analysis of Trial Results

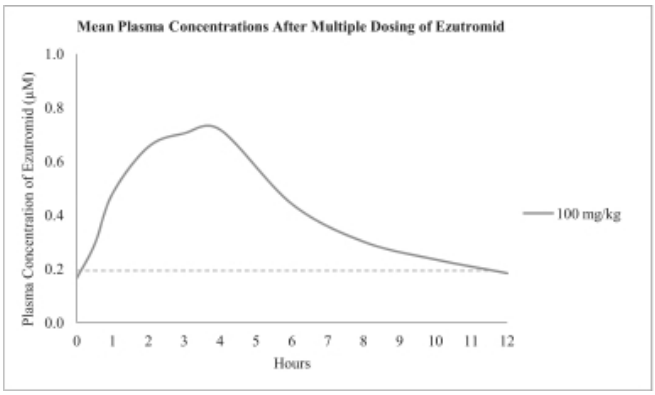
We observed the following results from this clinical trial:

- ***Ezutromid was Well Tolerated***. In both Part 1 and Part 2 of the clinical trial, ezutromid was well tolerated at all doses tested. The only observed treatment related adverse event was pale stools, which only occurred at the 200 mg/kg and 400 mg/kg dose levels. The pale stools were attributed to unabsorbed ezutromid passing through the gastrointestinal tract at these higher dose levels. All other adverse events were mild in severity and resolved without treatment.
- ***Higher Plasma Concentrations When Ezutromid Dosed with Food***. The dietary state of subjects in the clinical trial had a meaningful effect on systemic exposure. As illustrated in the figure below, after we administered a single dose of 200 mg/kg of ezutromid to subjects in the 200 mg/kg cohort of Part 1 of the clinical trial, the mean plasma concentration of drug in the blood over time, as determined by quantification of the area under the curve, in the subjects when they were in a fed state (n = 6) was approximately five times higher than the same subjects when they were in a fasted state (n = 5).



- ***Targeted Plasma Levels Achieved in All Subjects after Multiple Dosing***. When we administered 100 mg/kg doses of ezutromid twice a day for ten days, the steady state plasma concentration achieved in all subjects was greater than 0.2 μM (67 ng/mL), which was the concentration that corresponded to a 50% increase in utrophin protein levels in our preclinical studies described in more detail below. The mean blood plasma concentration of ezutromid in the 12 hours following administration of the final dose is illustrated in the figure below. However, there were differences among subjects, with the amount of time that each subject had plasma concentrations of utrophin protein greater than 0.2 μM

ranging from seven to 12 hours following dosing. Utrophin protein has a half-life of three to four weeks, and we believe that a few hours of exposure to ezutromid following regular dosing may lead to an accumulation of utrophin protein in muscle tissue over time. Subjects receiving 200 mg/kg doses of ezutromid twice a day for ten days did not achieve higher plasma concentrations of ezutromid than subjects receiving 100 mg/kg doses of ezutromid on this dosing schedule. As a result, we expect that the maximum dose of ezutromid in our future clinical trials will be 100 mg/kg.



- ***Stable Plasma Levels of Ezutromid When Administered Through Multiple Dosing***. When we administered 100 mg/kg doses of ezutromid twice a day for ten days with meals, all subjects achieved stable, or steady state, blood plasma concentrations of drug within three to five days after the beginning of dosing. However, we observed differences in plasma concentrations across subjects, which we believe resulted from varying levels of activity of CYP1A, a liver enzyme that metabolizes ezutromid, in different subjects.

Initial Phase 1b Clinical Trial in DMD Patients (Trial 02)

In May 2014, we completed an open label, ascending single and multiple oral dose Phase 1b clinical trial of ezutromid in patients with DMD. We believe this clinical trial was the first time a utrophin modulator drug had been administered to DMD patients. We conducted this clinical trial at four sites in the United Kingdom under approval from the MHRA and the Ethics Review Committee. We enrolled 12 boys with DMD who were between five and 11 years of age.

The primary objective of the clinical trial was to determine the safety and tolerability of single and multiple oral doses of ezutromid. The secondary objectives were to determine the single and multiple oral dose pharmacokinetics of ezutromid and its metabolites in patients with DMD. In addition, an exploratory objective of the clinical trial was to quantify potential systemic activity biomarkers.

We divided the patients into three cohorts of four boys each. Patients in each of the cohorts received different doses of ezutromid for 11 days. The patients in all of the cohorts were treated in a fed state. The clinical trial protocol provided for the administration of ezutromid within ten minutes after consuming a substantial meal. Patients in the first cohort received the following doses of ezutromid:

- a single 50 mg/kg dose on day one;
- 50 mg/kg doses administered twice per day on days two to ten; and
- a single 50 mg/kg dose on day 11.

Patients in the second cohort received the following doses of ezutromid:

- a single 100 mg/kg dose on day one;
- 100 mg/kg doses administered twice per day on days two to ten; and
- a single 100 mg/kg dose on day 11.

Patients in the third cohort received the following doses of ezutromid:

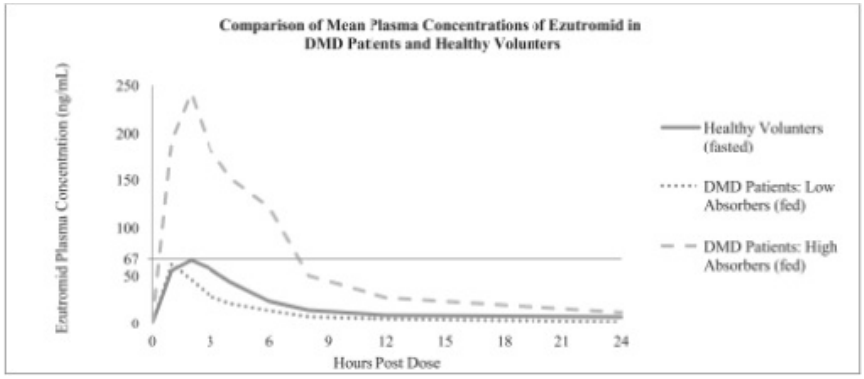
- a single 100 mg/kg dose on day one;
- 100 mg/kg doses administered three times per day on days two to ten; and
- a single 100 mg/kg dose on day 11.

Analysis of Trial Results

We observed the following results from this clinical trial:

- ***Ezutromid was Well Tolerated***. Ezutromid was well tolerated at all doses tested in this clinical trial with no serious adverse events reported. All reported adverse events were mild in severity and gastrointestinal in nature. In the opinion of the trial investigator, there were no clinically meaningful changes in physical examination, vital signs and hematology or biochemistry parameters in any of the patients. We also did not observe any issues with patient compliance.
- ***Patients had Variable Plasma Levels of Ezutromid; Possible Impact from Diet on Absorption of Ezutromid***. We observed variability among patients in all three cohorts in plasma concentrations of ezutromid after administering multiple daily doses for eleven days. As illustrated in the figure below, the mean blood plasma concentrations of two of the 12 DMD patients, who we refer to as high absorbers, exceeded the target level of 0.2 μM (67 ng/mL) for several hours following dosing. We determined this target level prior to conducting this clinical trial based on the composite results of our preclinical studies in tissue culture, or *in vitro* preclinical studies, and our preclinical studies in live animals, or *in vivo* preclinical studies, which indicated that this plasma concentration leads to an increase of approximately 50% in levels of utrophin protein. The mean plasma concentrations of the remaining ten patients, who we refer to as low absorbers, were less than this target level and similar to the levels achieved by fasted healthy volunteers in our completed Phase 1 clinical trial who had received a single 200 mg/kg dose of ezutromid. Nonetheless, we believe that the patients who did not achieve the target plasma level in the clinical trial may still have achieved a plasma level of ezutromid sufficient to modulate the production of utrophin and possibly result in a clinical benefit. This belief is based in part on the work of Professor Davies' research group, in which the continued expression of

utrophin protein in transgenic lines of an mdx mouse, even at levels just above those in a normal mdx mouse, had a meaningful, positive effect on muscle performance.



We believe that the similarity of ezutromid plasma levels between the majority of DMD patients in this Phase 1b clinical trial and fasted healthy volunteers in our completed Phase 1 clinical trial may be due to a complex absorption profile in DMD patients that results from patients following low fat, low calorie diets. DMD patients often follow such diets due to concerns over the consequences of long-term corticosteroid use and potential resulting weight gain. In addition, we believe that other DMD disease-related factors, such as abnormal gastrointestinal physiology, may impact the absorption profile of DMD patients.

- ***Patients Experienced a Reduction in CK and Other Enzyme Markers of Muscle Damage***. We observed a reduction compared to baseline in the enzymes CK, aspartate aminotransferase, or AST, and alanine aminotransferase, or ALT, in over 90% of the patients in the clinical trial during dosing with ezutromid. Other liver associated enzymes, gamma glutamyl transferase, alkaline phosphatase and albumin, showed no meaningful change from baseline over the same dosing period. The levels of CK, ALT and AST are typically low in healthy people. In DMD patients, however, damage to muscle fibers leads to the release of these enzymes from the muscle and accumulation in the blood. The mean reductions in CK, ALT and AST were statistically significant as compared to the baseline pre-dose levels ($p < 0.05$). We determine statistical significance based on a widely used, conventional statistical method that establishes the p-value of clinical results. Typically, a p-value of 0.05 or less represents statistical significance. Following the end of dosing, the levels of these enzymes increased toward pre-dose levels. In addition, the reduction in CK was consistent with the results of a preclinical *in vivo* study that we conducted in the *mdx* mouse model, described in more detail below, in which we observed a reduction in CK following single daily dosing of ezutromid. We did not observe a correlation between the dose level of ezutromid administered and the degree of change in the levels of these enzymes. Although this was not a placebo controlled study and there may be other factors that influenced the results, we believed at the time that the lower levels of CK, AST and ALT compared to baseline potentially indicated a reduction in muscle damage and may have been evidence of ezutromid activity. We consequently further evaluated this observed reduction in the enzyme markers of muscle damage in our subsequent Phase 1b modified diet trial. The results of the Phase 1b modified diet trial are described below.

Phase 1b Modified Diet Clinical Trial (Trial 03)

In August and September 2015, we reported results from our Phase 1b modified diet trial of ezutromid in patients with DMD. This trial evaluated the formulation of ezutromid, which we refer to as the "F3" formulation, and which was derived from the earlier clinical formulations of ezutromid that were evaluated in Trial 01 and Trial 02. We

conducted this clinical trial at four sites in the United Kingdom under approval from the MHRA and the Ethics Review Committee. We enrolled a total of 12 boys with DMD who were between five and thirteen years of age.

The primary objective of this clinical trial was to determine the pharmacokinetics of single and multiple oral doses of ezutromid in patients with DMD who followed specific dietary guidance that recommended balanced proportions of fat (30%), protein (25%) and carbohydrates (45%) and dosing with a glass of whole milk. We sought to achieve this dietary balance by requesting that patients, with support from research dietitians and the patients' legal guardians, consume a diet containing all of the major food groups, vitamins, minerals and dietary fiber, with a daily calorie intake that is appropriate for the age and activity level of each patient. The goal of this dietary guidance was to demonstrate an increase in the level of ezutromid in blood plasma compared to the blood plasma levels we observed among DMD patients in our initial Phase 1b clinical trial. The trial protocol included a number of secondary objectives, including evaluations of the safety and tolerability of single and multiple oral doses of ezutromid; the daily variability in the steady state pharmacokinetics of ezutromid; and the levels of CK as a potential biomarker of ezutromid activity.

We divided the patients into three cohorts of four patients each. The cohorts were randomized into three sequential 14-day treatment periods during which each patient in the clinical trial received ezutromid at a dose level of 1,250 mg, ezutromid at a dose level of 2,500 mg or a placebo. All doses were administered orally with 100 mL of whole milk and with patients having consumed either breakfast or an evening meal depending on the time of day. There was a wash out period, which is a period of time during which patients received no administration of the drug, of at least 14 days between each of the treatment periods. The clinical trial was blinded as to the order in which patients received the lower dose of drug, higher dose of drug or placebo. The patients in each cohort were dosed with either ezutromid or placebo as follows:

- a single dose on day one; and
- twice daily doses on days two to fourteen.

A follow-up safety visit was conducted twelve to fourteen days after administration of the final dose in the final treatment period. Each patient received specific dietary guidance after which there was a dietary run-in period of at least one week prior to the start of the first treatment period.

Analysis of Trial Results

We observed the following results from our Phase 1b modified diet trial:

- ***Modified Diet had a Positive Impact on Plasma Absorption***. In this trial, plasma absorption of ezutromid was increased in patients with DMD who followed specific dietary guidance that provided a balanced diet of fats, carbohydrates and proteins. Ten of the 12 patients achieved plasma exposure levels above 30 ng/mL for a mean of 14.0 hours in a 22-hour period on day 14 of the trial, with six of these patients achieving levels above 67 ng/mL for a mean of 8.2 hours in the same 22-hour period on day 14. Plasma levels of 30 ng/mL and 67 ng/mL correlate to an increase in utrophin levels of approximately 30% and 50%, respectively, based on our *in vitro* studies that were undertaken in myoblast cells from patients with DMD and myotubes from healthy individuals. The remaining two patients achieved maximum plasma exposure levels that exceeded 20 ng/mL. We believe that these two patients also achieved plasma exposure that may be sufficient to modulate the production of utrophin protein and possibly result in clinical benefit. The plasma exposure levels described above for all 12 patients were achieved after each received twice daily doses of 2,500 mg of ezutromid.

  The impact of adhering to the modified diet on the absorption of ezutromid was further evidenced when we compared the results of seven patients who participated in our initial Phase 1b clinical trial in 2014 and our Phase 1b modified diet trial. All of these seven patients had increased plasma levels in the Phase 1b modified diet trial as compared to plasma levels observed in our initial Phase 1b trial. The increase in plasma levels ranged from approximately 100% to nearly 300%.

- ***Higher Plasma Levels of Ezutromid were Observed on Day 14 Compared to Day 1 in the Majority of Patients***. In this trial, seven of the 12 patients had higher plasma levels when measuring plasma levels over time and calculating the area under the curve, or AUC, on day 14 compared to day 1. This accumulation of drug had not been observed in our previous Phase 1 clinical trial conducted in healthy volunteers or our initial Phase 1b clinical trial conducted in patients with DMD. We expect to evaluate the impact of longer-term dosing of ezutromid on plasma exposure in future clinical trials, including in our Phase 2 PhaseOut DMD clinical trial.
- ***Treatment with Ezutromid did not Alter CK Levels, an Enzyme Biomarker of Muscle Damage, Compared to Placebo***. We did not observe a change in the levels of the enzyme CK compared to baseline when patients were treated with 1,250 mg or 2,500 mg of ezutromid twice a day for 14 days compared to placebo. In our initial Phase 1b clinical trial, in which there was no placebo control, we observed a statistically significant reduction in CK levels compared to baseline when dosing patients with ezutromid. We believe that the results from our Phase 1b modified diet trial indicate that the reduction in CK levels we observed previously is likely not related to treatment with ezutromid. We plan to evaluate CK levels, as well as additional biomarkers, over a longer duration of exposure to ezutromid in future clinical trials.
- ***Ezutromid was Well Tolerated.*** Ezutromid was well tolerated at all doses tested in this clinical trial, with no serious adverse events reported. All reported adverse events were mild in severity and resolved prior to completion of the study. The most common adverse event was pale stools and this was reported by patients in the placebo group and each of the ezutromid treatment groups. In the opinion of the trial investigator, there were no clinically meaningful changes in physical examination, vital signs and hematology or biochemistry parameters in any of the patients. We also did not observe any issues with patient compliance.

Phase 1 Clinical Trial of Potential New Formulation of Ezutromid (Trial 04)

In August 2016, we reported top-line results from an open label, Phase 1 clinical trial of two new formulations of ezutromid. We conducted the trial at six sites in the United Kingdom under approval from the MHRA and the Ethics Review Committee.

The primary objective of the Phase 1 clinical trial was to determine the pharmacokinetics of multiple oral doses of the new formulations of ezutromid. The trial protocol included a number of secondary objectives, including evaluation of the safety and tolerability of single and multiple oral doses of the two formulations of ezutromid; to explore the effect of food to a fasting condition on the pharmacokinetics of ezutromid in healthy volunteers; and the daily variability in the steady state pharmacokinetics of ezutromid. The Phase 1 trial was divided into two parts. Part A evaluated two new formulations in healthy volunteers and Part B evaluated one of the two new formulations tested in Part A based upon the plasma concentration profile data and safety results. All doses were administered orally.

Part A

We enrolled a total of 16 healthy male volunteers between 18 to 55 years of age. There were two treatment periods each lasting five days: treatment period one evaluated one new formulation of ezutromid which we refer to as “F5” formulation, and treatment period two evaluated a different new formulation of ezutromid which we refer to as “F6” formulation.

In treatment period one, eight healthy male volunteers were administered the F5 formulation as follows:

- A twice daily dose of 3,000 mg on days one to three in a fed state;
- A twice daily dose of 6,000 mg on day four in a fed state;

- A single dose of 6,000 mg in the morning on day five in a fasted state; and
- A single dose of 6,000 mg in the evening on day five in a fed state.

In treatment period two, eight healthy male volunteers were administered the F6 formulation as follows:

- A twice daily dose of 2,000 mg on days one to three in a fed state;
- A twice daily dose of 4,000 mg on day four in a fed state;
- A single dose of 4,000 mg in the morning on day five in a fasted state; and
- A single dose of 4,000 mg in the evening on day five in a fed state.

The morning and evening doses were separated by approximately ten to twelve hours.

Based on the plasma concentration levels achived in healthy subjects and the safety data from Part A of the trial, the F6 formulation of ezutromid was selected for further evaluation in patients with DMD in Part B of the trial.

Part B

We enrolled a total of eight patients with DMD aged between 5 and 9 years of age and treated them with the F6 formulation of ezutromid that had been tested in Part A of the clinical trial. Patients received three ascending doses over three treatment periods:

- 250 mg twice a day for seven days;
- 500 mg twice a day for seven days; and
- 1,000 mg twice a day for seven days.

There was a wash out period of a minimum of seven days between treatment periods. Patients followed the same specific dietary guidance as we used in our Phase 1b modified diet clinical trial.

Analysis of trial results

We observed the following results from our Phase 1 clinical trial evaluating the new formulations of ezutromid:

- ***Healthy Volunteers Demonstrated Increased Plasma Levels With the F6 Formulation of Ezutromid.*** In healthy volunteers, the F6 formulation of ezutromid achieved an over ten-fold increase in plasma levels compared to the F3 formulation of ezutromid. This formulation was selected to progress into Part B of the trial and undergo evaluation in patients with DMD.
- ***DMD Patients Demonstrated Increased Plasma Levels of Ezutromid with F6 Formulation.*** At the 1,000 mg twice a day dose of the F6 formulation of ezutromid, the five evaluable patients achieved an average maximum plasma concentration of 390 ng/mL on day 7, the final day of dosing. By comparison, in our Phase 1b modified diet trial, a twice daily dose of 2,500 mg of the F3 formulation of ezutromid achieved an average maximum plasma concentration of 63 ng/mL on day 14, the final day of dosing. We believe both the F6 and F3 formulations of ezutromid will be able to modulate utrophin. However, we anticipate that only one of these formulations will be chosen to move forward in clinical development, based on the safety and efficacy data from PhaseOut DMD.
- ***Ezutromid was Generally Well Tolerated.*** Ezutromid was generally well tolerated at all doses tested in both the healthy volunteers and DMD patients, except for one DMD patient who exhibited changes in liver parameters and withdrew from the trial, despite showing no clinical symptoms. The finding was classified as a serious adverse event.

We plan to publish full results from our Phase 1 clinical trial evaluating new formulations of ezutromid in peer reviewed literature.

BioMarin Phase 1 Clinical Trial in Healthy Volunteers

In 2009, we assigned certain technology relating to our DMD program to BioMarin DMD Regulator Limited, or BioMarin. In 2010, BioMarin conducted a Phase 1 clinical trial of a prior formulation of ezutromid in 48 healthy adult volunteers. The clinical trial was conducted at a single site in the United Kingdom. BioMarin reported that ezutromid was well tolerated by the subjects in this clinical trial. Subjects in this trial achieved low systemic exposure of the drug, and there was variability in systemic exposure across subjects. Following this clinical trial of a prior formulation of ezutromid, BioMarin elected not to continue development of our assigned technology, citing pharmaceutical and pharmacokinetic challenges. In public statements, BioMarin indicated that it had concluded that the likelihood of achieving a therapeutic effect in DMD patients was highly unlikely. In 2010, BioMarin transferred the assets, and all commercialization rights, back to us. As described above, in our Phase 1 clinical trial of ezutromid in healthy volunteers, in which we administered ezutromid as a flavored aqueous suspension, we were able to achieve our target plasma concentrations in all subjects after multiple dosing.

Ongoing Phase 2 'PhaseOut DMD' Clinical Trial

We are conducting PhaseOut DMD, an open-label trial into which we plan to enroll approximately 40 ambulatory boys between their fifth and tenth birthday inclusive who have a genetically confirmed diagnosis of DMD. The enrolled patients must be on stable doses of corticosteroids for a minimum of six months. We began conducting the trial at sites in the United Kingdom after receiving approval from the MHRA in January 2016 and in the United States after our investigational new drug application was cleared by the FDA in April 2016. We commenced enrollment at trial sites in the United Kingdom in June 2016 and at trial sites in the United States in November 2016. Based on our current estimates, we expect to complete enrollment for PhaseOut DMD in the second quarter of 2017. We have also submitted an amendment to the PhaseOut DMD clinical trial protocol to the MHRA, UK Ethics Committee and the FDA to allow the trial to be extended beyond the intial 48 weeks of dosing and to include a new safety arm.

In PhaseOut DMD, approximately 30 patients will receive the F3 formulation of ezutromid at a dose level of 2,500 mg twice daily via oral administration, and approximately ten patients will receive the F6 formulation of ezutromid at a dose level of 1,000 mg twice daily via oral administration. All patients will receive dietary guidance, consistent with that provided in our Phase 1b modified diet trial, to ensure they are receiving balanced proportions of fat, proteins and carbohydrates. At the time of dosing, patients will consume whole milk and will have also recently eaten either breakfast or an evening meal, depending on the time of day. The trial protocol specifies that there should be a gap of between eight to twelve hours between the breakfast and evening meal doses.

We have designed the PhaseOut DMD trial to evaluate the activity and safety of ezutromid and utrophin modulation and it will consist of the following four parts:

- ***Screening and Baseline Stage***: A screening and baseline phase lasts up to 28 days for each patient. During this time, we take a number of baseline measurements. These include MRI analysis of upper leg muscle, a baseline muscle biopsy, blood samples for pharmacokinetic and enzyme biomarker measurements, and baseline measurements for functional tests, including the six minute walk test and the North Star Ambulatory Assessment.
- ***Treatment Stage***: The treatment phase for each patient lasts a total of 48 weeks. During the treatment stage, an MRI analysis will be conducted at 12, 24, 36 and 48 weeks of treatment. Blood samples for pharmacokinetic and enzyme biomarker analysis will be taken at 4, 8, 12, 24, 36 and 48 weeks of treatment. In addition, a portion of the patients will have a second muscle biopsy taken at week 24, with the remaining patients having their second biopsy at week 48. Functional tests will be performed at 12, 24, 36 and 48 weeks of treatment.

- ***Safety and Tolerability Follow-up Stage***: Each patient in the trial will have a 30-day safety and tolerability follow up. For trial patients who do not enroll in the extension phase, the safety and tolerability follow-up will occur after the 48 week treatment phase. Patients enrolling in the extension phase are expected to have a 30-day safety and tolerability follow-up following completion of the extension phase.
- ***Trial Extension Phase***: We have also submitted an amendment to the clinical trial protocol to the MHRA, UK Ethics Committee and the FDA to allow all patients in the trial to continue to be dosed with a similar assessment regimen of ezutromid. Subject to our receipt of regulatory approval for the extension, we expect that the extension phase will last until ezutromid either receives marketing approval in the relevant country or its development is discontinued. This extension phase will allow us to monitor safety and efficacy data related to longer-term dosing of the F3 and F6 formulations of ezutromid. The decision to submit the protocol amendment was made following a review of the safety and tolerability data from PhaseOut DMD by an independent data monitoring committee.
- ***Safety Arm:*** The amended protocol we submitted to the regulatory authorities and ethics committee to extend the trial also provides for the enrollment into PhaseOut DMD of patients who participated in prior clinical trials of ezutromid but who did not meet the inclusion criteria for PhaseOut DMD. All safety, tolerability and functional data from this arm of the trial will be distinct from analyses of the efficacy portion of PhaseOut DMD.

Clinical Trial Objectives

The primary objective of our PhaseOut DMD clinical trial is to investigate changes from baseline in leg muscle health using MRI. Reports in the peer reviewed literature have shown MRI has potential as a non-invasive biomarker to measure disease progression through measurement of changes in inflammation and fat infiltration in leg muscles. This trial will monitor disease progression after treatment with ezutromid by measuring changes from baseline in levels of muscle inflammation and fat infiltration in leg muscle during the course of the 48 week trial. We will also investigate if there are any relationships between changes in leg muscle MRI with blood plasma concentrations of ezutromid, which we will measure at baseline and over the course of the trial.

We also will investigate in our PhaseOut DMD clinical trial changes in utrophin expression in muscle and muscle fiber regeneration. The muscle biopsies taken during the trial will be used to investigate changes from baseline in utrophin protein expression and changes in muscle regeneration biomarkers.

The clinical trial is also expected to investigate a number of functional measures. These will include changes from baseline in distance walked during the six minute walk test, changes from baseline in the North Star Ambulatory Assessment, changes from baseline in a 10 meter run test, and changes from baseline in performance of upper limbs. We will also monitor changes in a variety of blood biomarkers related to muscle health, including the enzyme CK, during the trial.

We believe that these objectives collectively will enable us to better understand the potential benefits of long-term dosing with the F3 and F6 formulations of ezutromid on the progression of DMD in a pediatric population. We will also seek to understand if there are any potential relationships between changes in MRI, utrophin expression, muscle fiber regeneration and other assessments of muscle function. This trial will be the longest period of time that ezutromid has been dosed in patients and will increase the amount of safety and tolerability data for ezutromid.

We expect to report 24-week biopsy data in the first quarter of 2018 for all patients in PhaseOut DMD who provide a 24-week biopsy sample. We expect this group will consist of approximately 20 patients dosed with either the F3 formulation or F6 formulation of ezutromid. We plan to report this full 24-week biopsy data instead of reporting an earlier interim biopsy analysis on the first group of patients enrolled in the trial, which we previously expected to announce in the second quarter or third quarter of 2017. We believe this revised approach

will allow us to report more complete efficacy and safety data from PhaseOut DMD by evaluating a larger number of patients. We plan to analyze all 24-week biopsy data once all the samples have been taken. In addition, at the same time we expect to announce the 24-week biopsy data, we also expect to announce 24-week MRI and functional data analysis from all patients in the trial.

If we receive positive interim data from PhaseOut DMD, we plan to initiate a randomized, placebo controlled clinical trial of ezutromid. We anticipate that this trial will be designed with the potential to support accelerated regulatory approval in the United States and conditional approval in the European Union. We expect to provide an update on timing for the start of this randomized, placebo controlled clinical trial following the release of the 24-week interim dataset from PhaseOut DMD. In addition, a separate confirmatory clinical trial designed to support full regulatory approvals of ezutromid in major territories is also expected to be conducted.

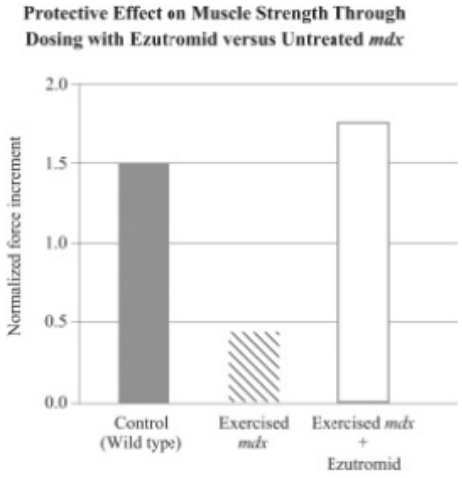
Ezutromid Preclinical Studies

We have conducted a broad preclinical program for ezutromid in collaboration with Professor Kay Davies and her research group at the University of Oxford. The preclinical program consists of *in vitro* and *in vivo* studies designed to support the potential of ezutromid to modulate the expression of utrophin protein. The following is a summary of key observations from studies completed to date:

- ***Increased Utrophin Levels in DMD Patient Derived Myoblast Cells***. We dosed *in vitro* muscle derived cells called myoblasts from DMD patients with ezutromid. After three days of dosing, we observed a two-fold mean increase in utrophin protein levels in these myoblast cells as compared to baseline levels.
- ***Increased Utrophin Protein Expression in Heart, Diaphragm and Other Skeletal Muscles in mdx Mouse***. We dosed *mdx* mice with ezutromid daily for 28 days. Following the 28 days of dosing, we observed increased mean utrophin protein levels in the diaphragm ($p<0.05$) and the heart ($p<0.01$) as compared to untreated *mdx* mice. We also observed an increase in utrophin protein levels in the tibialis anterior, or TA, and extensor digitorum longus, or EDL, skeletal muscles. We also observed a mean increase in utrophin messenger ribonucleic acid, or mRNA, which is the precursor to utrophin protein. We believe that the good systemic distribution of drug observed in this experiment is important for DMD therapies that aim to maintain ambulation and prolong life for DMD patients.
- ***Localized Utrophin Production at the Sarcolemma in mdx Mouse***. In the *mdx* mouse experiment described in the prior bullet, we observed an increase in utrophin protein in the TA and EDL skeletal muscles of *mdx* mice treated with ezutromid compared to untreated *mdx* mice as evidenced by an observable increase in the number of utrophin positive muscle fibers in these muscles. The increase in utrophin protein was localized at the sarcolemma, which is the required site of action for utrophin production in muscle. In a separate study in which we forced *mdx* mice to exercise, we observed a similar increase in utrophin positive muscle fibers in the diaphragm and the TA and EDL muscles, and an increase of utrophin levels within these muscle fibers, of *mdx* mice treated with ezutromid compared to untreated *mdx* mice. We believe that these results are noteworthy because DMD disease pathology is even more pronounced in the diaphragm and hind-limb muscles of the forced exercise *mdx* mice as compared to sedentary *mdx* mice.
- ***Reduction in Secondary Markers of DMD in mdx Mouse***. We dosed *mdx* mice with ezutromid daily for 28 days. In this study, we observed a mean 75% reduction in CK levels as compared to untreated *mdx* mice after 15 days, which is the time at which muscle degeneration is at a maximum in this model. We continued to observe lower mean CK levels in the treated *mdx* mice group after 28 days, at which point muscle degeneration stabilized. Plasma levels of CK, muscle regeneration, inflammation and fibrosis are secondary markers of DMD. We also observed a reduction in the mean level of muscle fiber regeneration in *mdx* mice treated with ezutromid compared to untreated *mdx* mice as evidenced by a reduction in the number of muscle fibers with centrally localized nuclei, which are biomarkers of regeneration. We believe this resulted from the continual expression of utrophin, which protected the

dystrophin deficient muscle fibers, and therefore reduced the amount of muscle regeneration. In addition, following treatment with ezutromid, we observed a mean reduction in overall skeletal muscle inflammation and fibrosis in the *mdx* mice treated with ezutromid compared to untreated *mdx* mice, which indicates a reduction in muscle fiber damage.

- ***Protection of Muscle Function in Forced Exercise mdx Mouse***. We dosed *mdx* mice with ezutromid daily for 28 days and forced the mice to exercise during this treatment period. As illustrated in the figure below, at the end of dosing the forced exercise *mdx* mice treated with ezutromid demonstrated a statistically significant mean increase in protection against exercise-induced forelimb weakness ($p<0.05$) compared to untreated forced exercise *mdx* mice. We measured forelimb weakness by the force increment required for the *mdx* mice to lose the strength to grip. The *mdx* mice treated with ezutromid exhibited forelimb strength comparable to that observed in the wild type control mice, which unlike *mdx* mice are not dystrophin deficient. The untreated *mdx* mice experienced a mean decrease in forelimb strength by the end of the 28 day study. Forcing the *mdx* mouse to exercise worsens the impact of DMD and we believe more closely approximates the pathology of human DMD patients.



- ***Target Plasma Concentration to Achieve a 50% Increase in Utrophin Levels***. The composite results from our *in vitro* and *in vivo* preclinical studies indicated that a plasma concentration of approximately 0.2 µM (67 ng/mL) leads to an increase of approximately 50% in levels of utrophin protein. These plasma concentration findings formed the basis of the target pharmacokinetic level that we have used in our clinical trials of ezutromid. As noted above, in the experiments performed by Professor Kay Davies, the continued expression of utrophin, even at levels just above those in a normal *mdx* mouse, had a meaningful, positive effect on muscle performance.

Our Pipeline of Future Generation Utrophin Modulators

We plan to apply and enhance our existing knowledge, experience and proprietary rights to maintain and expand our leadership in the field of utrophin modulation. Our co-founder and scientific advisor, Professor Kay Davies at the University of Oxford, discovered utrophin and then developed the concept of utilizing utrophin modulation as a treatment potentially applicable to all DMD patients. Our DMD program was founded to develop and commercialize drugs to treat DMD using this approach. Our intellectual property estate for ezutromid for the treatment of DMD includes composition of matter patents granted in major territories, including the United States and Europe. We plan to apply and enhance our existing knowledge, experience and proprietary rights to maintain and expand our leadership in the field of utrophin modulation. In addition to the F3 and F6 formulations of

ezutromid, we are currently pursuing novel, future generation utrophin modulators, some of which may have potential new utrophin-related mechanisms, that we are developing in collaboration with the University of Oxford.

We were previously pursuing internally developed second generation utrophin modulators that are structurally related to ezutromid, but designed to include improved pharmacokinetic properties and achieve higher plasma levels of drug at lower doses. However, in September 2016, we announced that the development of our second generation utrophin modulators would be placed on hold due to the substantial increase in ezutromid plasma levels achieved with the F6 formulation of ezutromid in our recently completed Phase 1 trial. We are now focusing on advancing the F3 and F6 formulations of ezutromid and our preclinical future generation utrophin modulators.

Strategic Alliance with the University of Oxford

In November 2013, as part of our program for the development of additional utrophin modulators, we formed a strategic alliance with the University of Oxford. Under this alliance, we acquired an exclusive option to license intellectual property that is generated as part of our research in utrophin modulation as part of the alliance. We announced in November 2015 a multi-year extension of the strategic alliance with the University of Oxford that will run until November 2019, with an option to extend it for a further 12 months. The goal of our collaboration with the University of Oxford is to identify and develop future generations of novel utrophin modulators that will include new mechanisms that could complement ezutromid and our second generation modulators. In December 2015, we reported achievement of the first research milestone as part of the collaboration with the nomination of two series of utrophin modulators for progression into lead optimization studies. These two series of compounds are structurally distinct from ezutromid, with one series having a potential new utrophin modulation mechanism that appears to be distinct from ezutromid.

Biomarker Program

We believe that the development of new biomarkers could play an important role in furthering our understanding of the potential benefits of utrophin modulators in treating DMD. A biomarker is a measurable biological or chemical change that is believed to be associated with the severity or presence of a disease or other physiological state of an organism. We expect our biomarkers will be related to the mechanism of utrophin modulation and will examine other aspects of muscle health, including inflammation and muscle fiber regeneration. Our biomarker program includes the following:

- quantifying numbers of utrophin positive fibers and distribution of utrophin protein in each fiber from muscle biopsies using immunohistochemistry;
- evaluating muscle biopsies to quantify numbers of regenerating fibers; and
- developing other serum biomarkers that will quantify muscle damage.

We are collaborating with the specialist biomarker development company Flagship Biosciences Inc., or Flagship, on the development of automated, digital analysis tools to allow for the precise and reproducible measurement of utrophin expression and levels of muscle fiber regeneration. We presented interim results from this research at the 21st International Congress of the World Muscle Society in October 2016. We believe that the development of these tools will have an important role in helping to further our understanding of the potential benefits of utrophin modulator therapies, including ezutromid, and we expect to use these tools in analyzing the muscle biopsies in our PhaseOut DMD clinical trial. Our collaboration with Flagship builds on a manual quantification approach that we developed with research groups at the Institute of Child Health at University College London. Data from our collaboration was published in the peer reviewed literature in March 2016.

***Clostridium difficile* Infection Overview**

Clostridium difficile Infection is a bacterial infection of the colon that produces toxins causing inflammation of the colon and severe diarrhea. CDI can also result in more serious disease complications, including pseudomembranous colitis, bowel perforation, toxic megacolon and sepsis. CDI represents a serious healthcare issue in hospitals, long-term care homes and, increasingly, in the wider community. We estimate there are over one million cases of CDI each year in the United States and Europe, based on an epidemiology report on CDI that was published in 2015 by Decision Resources, a healthcare research and consulting company. In addition, CDI is responsible for approximately 29,000 deaths per year in the United States, according to a study published in the *New England Journal of Medicine* in 2015. A separate study published in 2012 in *Clinical Microbiology and Infection*, a peer reviewed journal published by the European Society of Clinical Microbiology and Infectious Diseases, indicated that CDI may be underdiagnosed in approximately 25% of cases. A study published in *The Journal of Hospital Infection*, a peer reviewed journal published by the Healthcare Infection Society, reported that CDI is two to four times more common than hospital associated infections caused by methicillin-resistant *Staphylococcus aureus*, a bacterium frequently associated with such infections. The Healthcare Cost and Utilization Project, a family of databases developed through a federal-state-industry partnership sponsored by the Agency for Healthcare Research and Quality of the U.S. Department of Health and Human Services, reported an approximate 3.5 fold increase in hospital stays associated with CDI between 2000 and 2008. The economic impact of CDI is significant. A study published in 2012 in *Clinical Infectious Diseases* estimated that acute care costs total $4.8 billion per year in the United States alone.

CDI originates from a bacterium known as *Clostridium difficile*, or *C. difficile*. *C. difficile* sometimes can be a harmless resident of the gastrointestinal tract. The complex community of microorganisms that make up the natural gut flora usually moderates levels of *C. difficile*. The natural gut flora are an essential part of the normal function of the gastrointestinal tract and also have wide implications to human health, such as the proper function of the immune system. CDI typically develops following the use of broad spectrum antibiotic agents that can cause widespread damage to the natural gut flora and allow overgrowth of *C. difficile*. Hypervirulent *C. difficile* strains have also emerged and are frequently associated with more severe disease. In the United States, the hypervirulent strain, ribotype 027, accounts for approximately one-third of all CDI cases.

The primary clinical issue with CDI is disease recurrence. This is in contrast to other bacterial threats for which drug resistance is the principal concern. According to an article published in 2012 in the peer reviewed journal *Clinical Microbiology and Infection*, up to 25% of patients with CDI suffer a second episode of the infection. The risk of further recurrence rises to 65% after a patient suffers a third episode of CDI. In addition, each episode of recurrent disease is associated with greater disease severity and higher mortality rates. Recurrent disease is associated with an increased burden on the healthcare system.

In 2013, the CDC highlighted CDI as one of three pathogens that pose an immediate public health threat and require urgent and aggressive action. In 2012, the Generating Antibiotics Incentives Now Act provisions of the FDA Safety and Innovation Act, or GAIN, became law. The goal of GAIN is to encourage the development of new antibiotics that treat specific pathogens, including *C. difficile*, which cause serious and life threatening infections.

Current CDI Treatments

Existing treatment options for CDI are limited. The current standard of care for CDI is treatment with vancomycin or off label use of metronidazole, both of which are broad spectrum antibiotics. Although these antibiotics reduce levels of *C. difficile*, both also cause significant collateral damage to the gut flora as a result of their broad spectrum of activity. This collateral damage to the gut flora leaves patients vulnerable to recurrent CDI. A review published in 2012 in the peer reviewed journal *International Journal of Antimicrobial Agents* reported recurrence rates of 24.0% for vancomycin and 27.1% for metronidazole. Metronidazole is frequently used in mild or moderate cases of CDI and has been associated with a number of side effects. A narrower

spectrum antibiotic fidaxomicin was approved in the United States and the European Union, but it has not been shown to be superior to vancomycin in the treatment of patients with the hypervirulent strain ribotype 027. In October 2016, the FDA approved bezolotoxumab, a monoclonal antibody for use in conjunction with an antibiotic in patients who have a high risk of disease recurrence. Bezolotoxumab binds to toxin B, one of the toxins produced by the *C. difficile* bacteria, to neutralize its effects.

Ridinilazole for the Treatment of CDI

We are developing ridinilazole as an orally administered small molecule antibiotic for the treatment of CDI. Ridinilazole is designed to selectively target *C. difficile* bacteria without causing collateral damage to the gut flora and thereby reduce CDI recurrence rates. The active ingredient in ridinilazole is a bis-benzimidazole tetrahydrate. We believe, based on preclinical studies conducted to date, that ridinilazole is part of a novel structural class of antibiotics that is distinct from the major classes of marketed antibacterials.

In November 2015, we reported top-line results from our double blind, randomized, active controlled Phase 2 clinical trial that evaluated ridinilazole compared to the current standard of care, vancomycin, for the treatment of CDI. The Phase 2 clinical trial exceeded its primary endpoint of non-inferiority, with ridinilazole achieving statistical superiority over vancomycin in sustained clinical response, or SCR. The statistical superiority was driven by a large numerical reduction in recurrent disease compared with vancomycin. We subsequently reported that data from our Phase 2 clinical trial also showed ridinilazole to be highly preserving of the gut microbiome compared to patients who received vancomycin and experienced substantial damage to the gut microbiome which for many patients persisted during the 30-day post-treatment period. Ridinilazole was well tolerated at all doses tested in our completed Phase 1 and Phase 2 clinical trials. Activities to prepare ridinilazole for Phase 3 clinical trials continue, and we plan to commence these trials in the first half of 2018. We are currently exploring funding options for the Phase 3 clinical development program for ridinilazole and various options to maximize the value of ridinilazole, including potentially entering into a collaboration with a third party or securing meaningful non-dilutive funding from government entities and philanthropic, non-government and not for profit organizations.

In February 2015, we initiated an exploratory, open label, active controlled Phase 2 clinical trial evaluating ridinilazole compared to fidaxomicin for the treatment of CDI. Enrollment and dosing of patients is complete, and we expect to report top-line results from this clinical trial in the second quarter of 2017. We expect the results of this clinical trial will help to inform the commercial positioning of ridinilazole. The FDA has designated ridinilazole as a qualified infectious disease product, or QIDP. The QIDP incentives are provided through GAIN. The QIDP designation provides for priority review by the FDA, eligibility for "fast track" status and extension of statutory exclusivity periods in the United States for an additional five years upon FDA approval of the product for the treatment of CDI. The FDA granted fast track designation to ridinilazole in July 2015.

Ridinilazole Clinical Development

Phase 1 Clinical Trial in Healthy Volunteers

In 2013, we completed a randomized, partially blind, placebo controlled Phase 1 clinical trial of ridinilazole in healthy volunteers. We conducted this clinical trial at a single site in the United Kingdom under approval from the MHRA and the Ethics Review Committee. We enrolled 56 healthy male subjects in the clinical trial who were between 18 and 55 years of age. The primary objective of the clinical trial was to determine the safety and tolerability of single and multiple ascending oral doses of ridinilazole. The secondary objectives included determining the single and multiple oral dose pharmacokinetics of ridinilazole, assessing the effect of food on systemic exposure of ridinilazole and assessing the effect of multiple oral doses of ridinilazole on gut flora.

We conducted the clinical trial in two parts. Part 1 consisted of an ascending single dose study and a food effect evaluation study. In Part 1, we evaluated a total of 40 subjects, divided into the following six cohorts:

- four fasted subjects, randomized for three subjects to receive a single 2 mg dose of ridinilazole and one subject to receive placebo;
- four fasted subjects, randomized for three subjects to receive a single 20 mg dose of ridinilazole and one subject to receive placebo;
- eight fasted subjects, randomized for six subjects to receive a single 100 mg dose of ridinilazole and two subjects to receive placebo;
- eight fasted subjects, randomized for six subjects to receive a single 400 mg dose of ridinilazole and two subjects to receive placebo;
- eight fasted subjects, randomized for six subjects to receive a single 2,000 mg dose of ridinilazole and two subjects to receive placebo; and
- eight subjects, randomized for six subjects to receive a single 1,000 mg dose of ridinilazole under fasted conditions and a single 1,000 mg dose under fed conditions, and two subjects to receive two single doses of placebo on the same dosing schedule. The doses under fed and fasted conditions were separated by a minimum of six days.

Part 2 of the clinical trial consisted of a multiple dose study. In Part 2, we evaluated a total of 16 subjects, who were divided into the following two cohorts:

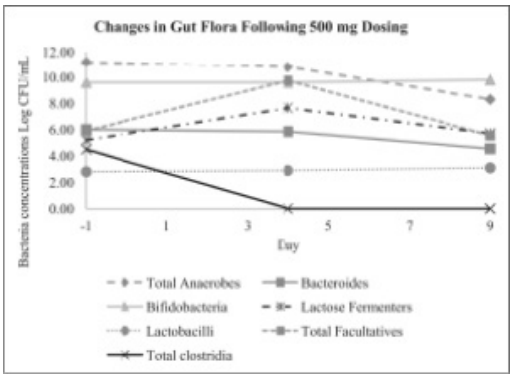
- eight subjects randomized for six subjects to receive 200 mg doses of ridinilazole twice per day for nine days with a single final dose on day ten and two subjects to receive placebo on the same dosing schedule; and
- eight subjects randomized for six subjects to receive 500 mg doses of ridinilazole twice per day for nine days with a single final dose on day ten and two subjects to receive placebo on the same dosing schedule.

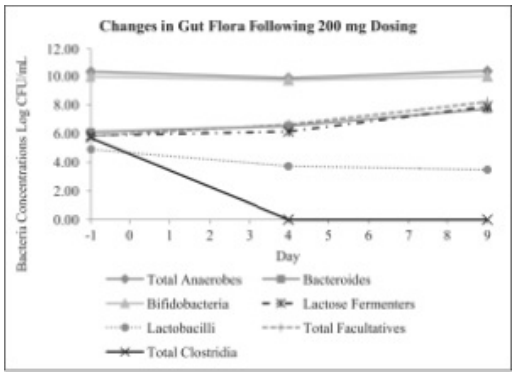
Analysis of Trial Results

We observed the following results in this clinical trial:

- ***Ridinilazole was Well Tolerated.*** Ridinilazole was well tolerated at all doses tested in the clinical trial. The incidence of adverse events in the clinical trial was low for patients treated with ridinilazole and comparable to the incidence of adverse events for patients receiving placebo. The majority of the adverse events that were considered to be possibly related to ridinilazole were classified as gastrointestinal disorders and were mild in severity and resolved without intervention. One patient withdrew from the clinical trial after suffering from appendicitis on day one. The trial investigator determined this serious adverse event was unlikely to be related to treatment with ridinilazole.
- ***Ridinilazole was Retained in the Gastrointestinal Tract.*** Ridinilazole was targeted to the gastrointestinal tract, which is the site where CDI occurs in the body. Systemic exposure was close to or below the level of detection in both fed and fasted subjects.
- ***Ridinilazole was Highly Selective for Total Clostridia Bacteria with Minimal Impact on Other Natural Gut Flora.*** We measured levels of bacteria in fecal samples from Part 2 of the clinical trial for gut flora composition on the day prior to commencement of dosing and on days four and nine of drug administration during the clinical trial. As illustrated in the figure below, in both the 200 mg and 500 mg dose cohorts, median levels of key bacteria groups that comprise the natural gut flora remained relatively constant during this period and did not fluctuate substantially from baseline. The one exception was the total clostridia bacterial group. The counts of total clostridia decreased from the

baseline level to zero by day four of dosing and remained at zero on day nine of dosing. *C. difficile* is a member of the total clostridia group. We did not detect any *C. difficile* viable cells or spores in the fecal samples of any of the healthy volunteer subjects at any point during the clinical trial. Bacteria levels are shown in the figure below on a logarithmic scale, which condenses the wide range of values to a format showing the relative differences in values. We believe these data, which are consistent with the data from our preclinical studies, support the highly selective antibiotic effect of ridinilazole.





Phase 2 Clinical Trial in Patients with CDI

In November 2015, we reported top-line results from our randomized, double blind, active controlled, multicenter, Phase 2 clinical trial of ridinilazole in patients with CDI, and we presented additional data during 2016. We have referred to this as our Phase 2 proof of concept clinical trial and as "CoDIFy."

We conducted this clinical trial at approximately 35 sites in the United States and Canada. The trial was conducted under an Investigational New Drug Application, or IND, that we submitted to the FDA in January 2014. We enrolled a total of 100 patients between 18 to 90 years of age. The trial randomized patients in a one-to-one ratio to receive either a 200 mg dose of ridinilazole administered twice per day for ten days or a 125 mg dose of vancomycin administered four times per day for ten days. Patients who received ridinilazole were also administered a placebo twice a day for ten days to ensure the trial remained blinded.

The primary objective of this clinical trial was to evaluate the efficacy of ten days of dosing with ridinilazole compared to treatment with vancomycin. The primary efficacy endpoint was non-inferiority on sustained clinical response, or SCR, which is defined as clinical cure based on the resolution of diarrhea at the test of cure, or TOC, visit on day 12 and no recurrence of CDI within 30 days after the end of treatment. The secondary efficacy endpoints were investigator assessed clinical response at the TOC visit and rate of recurrence of CDI within 30 days after the end of treatment. Secondary objectives of this clinical trial were the assessment of the safety and tolerability of ten days of dosing of ridinilazole compared to vancomycin, the plasma and fecal concentrations of ridinilazole in patients with CDI who received ridinilazole and the health status of CDI patients who received ten days of treatment of ridinilazole compared to patients who received ten days of treatment of vancomycin. We also assessed the impact of ridinilazole on the gut flora of patients in the clinical trial as one of a number of exploratory objectives.

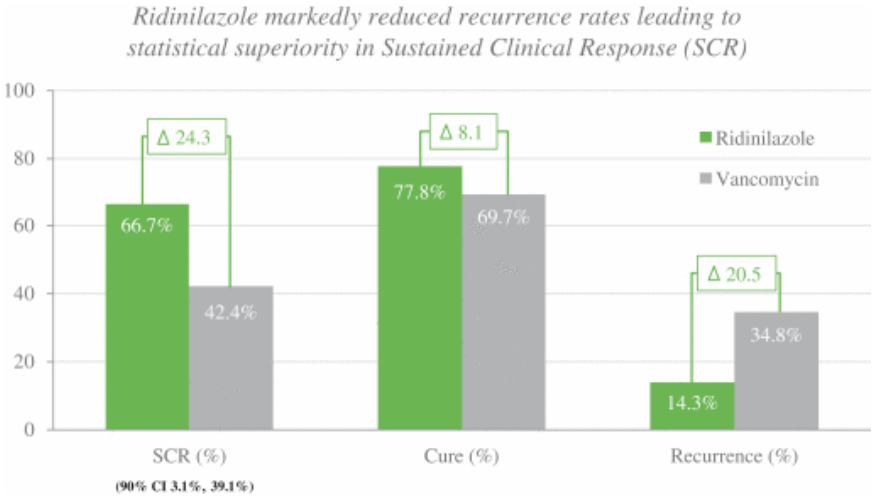
Analysis of Results

We observed the following results in our Phase 2 proof of concept trial:

- ***Ridinilazole Demonstrated Statistical Superiority Over Vancomycin.*** Our Phase 2 proof of concept trial met its primary endpoint with ridinilazole achieving a SCR rate of 66.7% compared to 42.4% for

vancomycin (non-inferiority margin of 15%, p=0.0004). This represented statistical superiority of ridinilazole over vancomycin using the pre-specified 90% confidence interval. The primary analysis was conducted on the modified intent-to-treat, or mITT, population (36 patients dosed with ridinilazole, 33 patients dosed with vancomycin) that comprised patients with CDI confirmed by the presence of free toxin in feces. The results of the mITT population were consistent with the intent-to-treat, or ITT, population (50 patients dosed with ridinilazole, 50 patients dosed with vancomycin) and the per protocol, or PP, population (31 patients dosed with ridinilazole, 25 patients dosed with vancomycin). We also observed a generally consistent trend to improved SCR with ridinilazole across subgroups at higher risk of recurrence, including the elderly, patients who were on concomitant antibiotics at the start of treatment and patients with a prior history of CDI.

- ***Ridinilazole Demonstrated a Large Reduction in Rates of Recurrence Compared to Vancomycin.*** We observed that the statistical superiority in SCR with ridinilazole compared to vancomycin was driven by a large numerical reduction in rates of disease recurrence. Clinical cure rates at the end of ten days of treatment were similar, with ridinilazole achieving a rate of 77.8% compared to 69.7% for vancomycin, but ridinilazole achieved a recurrence rate of 14.3% compared to 34.8% for vancomycin during the 30-day post-treatment period.



- ***Ridinialzole Preserved the Gut Microbiome.*** Stool samples were obtained from 82 patients enrolled in the Phase 2 clinical trial to evaluate the efficacy of ridinilazole compared to vancomycin. These samples were analyzed on study entry, day five and day ten of treatment, day 25 and day 40 post-entry and at the time of any recurrence for five specific bacterial groups associated with a healthy gut microbiome (*Bacteroides*, *Prevotella*, *Enterbactericeae*, *C. coccoides* and *C. leptum*) and also for total bacteria present. We observed that patients treated with vancomycin had a significant decrease ($p<0.001$) in four of the five bacterial groups (*Bacteroides*, *Prevotella*, *C. coccoides* and *C. leptum*) at day five and day ten, and a significant decrease in total bacteria. Patients treated with ridinilazole did not have a significant decrease in these specific bacterial groups nor the total bacteria. Moreover, we observed the initial evidence of recovery of these key bacterial groups in some patients treated with ridinilazole. We believe that these data provide evidence that ridinilazole is able to preserve a healthy gut microbiome during treatment for CDI and that the recovery of the key bacterial groups contributed to the large numerical reduction in disease recurrence we observed in the trial results.

- ***Ridinilazole was Retained in the Gastrointestinal Tract.*** Ridinilazole was targeted to the gastrointestinal tract, which is the site where CDI occurs in the body. Systemic exposure was close to

or below the level of detection in patients with CDI, with plasma concentrations very similar to those observed in our Phase 1 clinical trial in healthy volunteers.

- ***Ridinilazole Reduced Biomarkers of Inflammation.*** We measured levels of two key markers of inflammation, calprotectin and lactoferrin, in feces collected from the 69 patients who comprised the mITT group. The samples analyzed were collected at the time of randomization (prior to initiation of treatment), at day five and at day ten. We observed that ridinilazole and vancomycin reduced concentrations of calprotectin and lactoferrin by similar levels when analyzing the results for all patients. We also observed that a subset of patients with severe CDI had a greater reduction in levels of calprotectin and lactoferrin when treated with ridinilazole compared to vancomycin. We believe these data indicate that ridinilazole is associated with a greater reduction in inflammatory markers compared to vancomycin in patients with severe CDI.
- ***Ridinilazole was Well Tolerated.*** Ridinilazole was generally well tolerated. The overall rate of adverse events and serious adverse events reported in the ridinilazole and vancomycin treatment arms were comparable.

Ongoing Phase 2 Exploratory Clinical Trial of Ridinilazole Compared to Fidaxomicin

We have completed treatment in our randomized, open label, active controlled, multicenter Phase 2 clinical trial evaluating ridinilazole compared to fidaxomicin for the treatment of CDI. This clinical trial is designed to generate data comparing ridinilazole to fidaxomicin, a CDI antibiotic launched in 2011, and we expect the results of this clinical trial will help to inform the commercial positioning of ridinilazole. We conducted this clinical trial at sites in the United Kingdom, Europe and the United States, enrolling approximately 30 patients between 18 and 90 years of age. We randomized patients in a one-to-one ratio to receive either a 200 mg dose of ridinilazole administered twice per day for ten days or a 200 mg dose of fidaxomicin administered twice per day for ten days. We expect to report top line data from this clinical trial in the second quarter of 2017.

The primary efficacy objective of clinical this trial is to determine the safety and tolerability of ten days of dosing with 200 mg of ridinilazole compared to dosing with 200 mg of fidaxomicin. The secondary objectives of the clinical trial are to assess the following:

- the plasma pharmacokinetics of ridinilazole in patients with CDI;
- the qualitative and quantitative effect of ridinilazole and fidaxomicin on gut flora;
- the plasma, urine and fecal concentrations of ridinilazole and its metabolites; and
- the efficacy of ten days of dosing with ridinilazole compared to fidaxomicin for the treatment of CDI.

The measurement of efficacy will be based on investigator assessed clinical response at the TOC visit, with clinical cure defined as resolution of diarrhea while on treatment and maintained at the TOC visit, and sustained clinical response, defined as clinical cure at the TOC visit and no recurrence of CDI within 30 days after the end of treatment.

Phase 3 Clinical Trial Program

In February 2017, we outlined our plans for the Phase 3 development program of ridinilazole following an end of Phase 2 meeting with the FDA and a scientific advice process with the EMA. We expect to conduct two Phase 3 clinical trials evaluating ridinilazole compared to the standard of care antibiotic, vancomycin, with each trial expected to enroll approximately 700 patients with CDI. The primary endpoint of the Phase 3 clinical trials is expected to be superiority in SCR. Other planned endpoints include health economic outcome measures. The Phase 3 clinical trial designs are consistent with the successful proof of concept Phase 2 clinical trial of ridinilazole. Activities to prepare ridinilazole for Phase 3 clinical trials continue, and we plan to commence these trials in the first half of 2018. However, we do not expect to be able to complete these trials without significant

additional capital. We are currently exploring funding options for the Phase 3 clinical development program for ridinilazole and various options to maximize the value of ridinilazole, including potentially entering into a collaboration with a third party or securing meaningful non-dilutive funding from government entities and philanthropic, non-government and not for profit organizations.

CDI Preclinical Data

In a range of preclinical studies, ridinilazole demonstrated an encouraging profile as a potential antibiotic for the treatment of initial CDI and reduction of CDI recurrence. The following is a summary of key observations from these studies:

- ***Potency Against C. difficile***. We screened ridinilazole *in vitro* against panels of *C. Difficile* clinical isolates from the United States and the United Kingdom. In these studies, ridinilazole displayed a potent bactericidal effect against all clinical isolates of *C. difficile*, including hypervirulent strains, such as ribotype 027. Ridinilazole was more potent than both vancomycin and metronidazole, and was either equally potent to, or more potent than, fidaxomicin. We have also tested ridinilazole against a panel of *C. difficile* clinical isolates that maximize the diversity of resistance to key classes of commonly used antibiotics. Ridinilazole did not display evidence of cross resistance with other classes of key antibiotics in common use.
- ***Targeted Spectrum of Activity***. We conducted *in vitro* testing of ridinilazole, vancomycin, metronidazole and fidaxomicin against a wide panel of bacteria that are commonly found in the gut flora and are necessary for normal function of the gastrointestinal tract and also have wide implications on human health, such as the proper function of the immune system. As illustrated in the figure below, in this study ridinilazole had a minimal antibiotic effect against these beneficial bacterial groups. Ridinilazole also displayed higher selectivity for *C. difficile* in this study as compared to vancomycin, metronidazole and fidaxomicin and published data for cadazolid, an antibiotic that is currently in Phase 3 clinical development by another company. *In vitro* potency is measured by determining the concentration of a drug (in micrograms per liter) needed to inhibit the growth of 90% of the bacterial strains being tested, referred to as a MIC_{90} measurement. A high number, typically higher than 256, indicates a weak antimicrobial effect, and a low number, typically less than eight, indicates a potent antimicrobial effect. We believe that the targeted spectrum of activity for ridinilazole seen in this study compared to the relatively broad spectrum of activity of other antibiotics indicates the potential for ridinilazole to selectively target *C. difficile* bacteria without causing collateral damage to the gut flora and thereby reduce CDI recurrence rates.

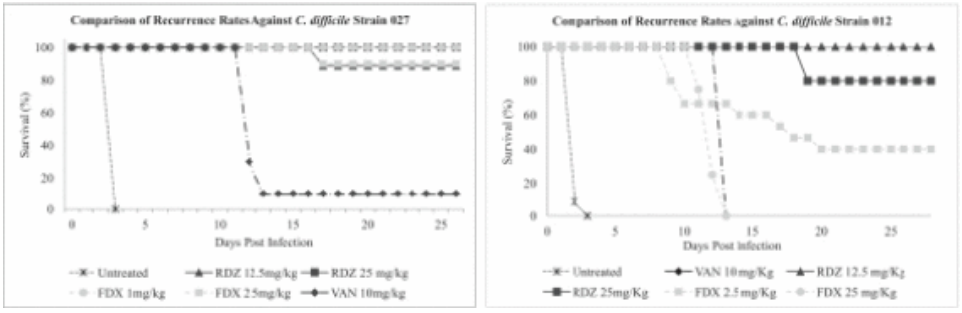
Profile of Selectivity of Ridinilazole vs. Other CDI Antibiotics

Key Bacterial Groups	Spectrum of Activity – MIC_{90} (µg/mL)				
	RDZ	MTZ	VAN	FDX	CAD*
Bacteroides spp.	>512	2	128	>512	4
Bifidobacterium spp.	>512	128	1	0.125	0.5
Lactobacillus spp.	>512	>512	>512	>512	-
Eggerthella lenta	>512	0.5	4	≤0.03	0.5
Peptostreptococcus spp.	64	1	0.5	≤0.03	-
Staphylococcus aureus	>512	>512	1	16	1.0

Antibiotic effect: Weak; Medium; Potent

RDZ: Ridinilazole VAN: Vancomycin CAD: Cadazolid
MTZ: Metronidazole FDX: Fidaxomicin

- ***Protection Against CDI Recurrence***. In a hamster model, we infected one group of hamsters with the hypervirulent CDI strain ribotype 027 and a second group of hamsters with a second CDI strain ribotype 012. In the United States, the hypervirulent CDI strain ribotype 027 accounts for approximately one third of all CDI cases. We then treated hamsters from each of the two infected groups with different doses of ridinilazole, vancomycin and fidaxomicin for five days. We evaluated disease recurrence over the 21 days following treatment. In this hamster model, a hamster fatality within the first five days is a result of initial *C. difficile* infection, while a fatality from day six to day 25 is a result of recurrent disease. As illustrated in the figure below, the hamsters from both infected groups that were treated with two different doses of ridinilazole had survival rates of 90% to 100% against strain ribotype 027 and 80% to 100% against strain ribotype 012. These survival rates were higher than hamsters treated with vancomycin (0% to 10% survival rates) for both CDI strains, comparable to hamsters treated with two different doses of fidaxomicin against strain ribotype 027 (90% to 100% survival rates) and higher than hamsters treated with two different doses of fidaxomicin against strain ribotype 012 (0% to 40% survival rates). All infection control hamsters received placebo and died by the second day following infection.



- ***Inhibition of Sporulation***. In the *in vitro* testing of ridinilazole described above, we treated *C. difficile* cells with different concentrations of ridinilazole and measured the percentage of spores formed 96 hours after treatment. Untreated cells had a 100% conversion rate into *C. difficile* spores, which are the dormant protected form of the bacteria, after 96 hours. In this study, treatment with ridinilazole

resulted in a meaningful reduction in spore count compared with untreated cells against all strains of *C. difficile* tested. We believe the reduction in sporulation may benefit rates of recurrent disease as the spores are highly resistant to standard cleaning practices and lead to increased risks of environmental persistence and disease transmission.

- ***Reduction in Toxin and Inflammation Levels***. In an *in vitro* study, Caco-2 cells, a type of cell found in the colon of humans commonly used in studies of intestinal function, were exposed to *C. difficile* and then treated with ridinilazole, metronidazole and vancomycin or were untreated to act as a control. Following treatment with ridinilazole, toxin A levels were reduced by 91%, toxin B was not detected and IL-8 levels were reduced by 74%. Metronidazole and vancomycin had minimal effect on toxin A or B concentrations, and IL-8 concentrations were similar to control. Toxins A and B are produced by *C. difficile* to elicit an inflammatory response, including IL-8 release, which results in the symptoms of the disease including severe diarrhea. We believe that these data indicate that ridinilazole has the potential to reduce the severity of disease symptoms and that it has the potential to be more effective than current treatment options.
- ***Concomitant Antibiotic Use***. In an *in vitro* bacterial culture study, we administered ridinilazole in combination with selected other antibiotics. In this study, concomitant use of antibiotics had neither a synergistic nor an antagonistic effect on the MIC_{90} values of ridinilazole against the *C. difficile* strains tested. We believe these results indicate that concomitant use of other antibiotics will not diminish the potency of ridinilazole. We believe this is an important finding because a significant portion of CDI patients receive antibiotic treatment for persistent or new infections.
- ***Low Propensity for Resistance***. In an *in vitro* study, we treated *C. difficile* bacteria with ridinilazole and assessed the number of resistant bacteria at the end of treatment. We repeated this process multiple times, with each cycle referred to as a serial passage. We observed that use of ridinilazole resulted in a low frequency of spontaneous mutation and no resistance after 14 serial passages of treatment. We have also evaluated ridinilazole mutant prevention concentration, or MPC, a measure evaluating the ability of an antibiotic to minimize the development of resistant organisms, against *C. difficile* clinical isolates. *In vitro* results show that ridinilazole has low MPC values against these isolates, providing further evidence supporting ridinilazole's profile for low resistance development.
- ***Ridinilazole Arrests Cell Division***. In an *in vitro* study, we treated *C. difficile* bacteria with ridinilazole and assessed its effects on killing the bacteria. The study revealed that ridinilazole halts *C. difficile* cell division, characterized by a significant increase in the length of *C. difficile* cells and an absence of division septum formation.

Our Collaborations and Funding Arrangements

Sarepta Therapeutics, Inc.

In October 2016, we entered into an exclusive license and collaboration agreement with Sarepta, pursuant to which we granted Sarepta the exclusive right to commercialize products in our utrophin modulator pipeline in the European Union, Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States, which we refer to as the licensed territory. Such products include ezutromid and our future generation of small molecule utrophin modulators, which we refer to as licensed products. We also granted Sarepta an option to expand the licensed territory to include certain specified countries in Central and South America. We retain commercialization rights in the rest of the world.

Financial Terms

Under the terms of the license and collaboration agreement, we received an upfront payment of $40.0 million from Sarepta. In addition, we are eligible to receive up to $42.0 million from Sarepta in specified development milestones for ezutromid, including a $22.0 million milestone, payable on or after April 1, 2017, following the

first dosing of the last patient in PhaseOut DMD, and up to $150.0 million from Sarepta in specified regulatory milestones related to ezutromid in the licensed territory. We are also eligible to receive up to $65.0 million in specified development milestones and up to $225.0 million in specified regulatory milestones from Sarepta for our future generation small molecule utrophin modulators in the licensed territory. In addition, we are also eligible to receive up to $330.0 million from Sarepta in specified sales milestones on a product-by-product basis, as well as tiered, escalating royalties ranging from a low to high teens percentage of net sales on a product-by-product basis in the licensed territory. The royalties are subject to potential reductions, including for a specified portion of royalty payments that Sarepta may become required to pay under any third-party license agreements, subject to a maximum royalty reduction.

Research and Development Obligations

Under the license and collaboration agreement, we have agreed to collaborate with Sarepta on the research and development of the licensed products pursuant to a joint development plan through a joint steering committee comprised of an equal number of representatives from each party. Sarepta has the final decision making authority with respect to commercialization decisions of the licensed products in the licensed territory. If the joint steering committee elects not to pursue development of a second generation or future generation small molecule utrophin modulator candidate, then we may engage, under certain circumstances, in the development of such candidate for commercialization outside of the licensed territory and outside of the agreement, subject to Sarepta's option, exercisable at Sarepta's discretion and only available to Sarepta under certain specified circumstances, to bring such candidate under the license and collaboration agreement.

Under the license and collaboration agreement, we are solely responsible for all research and development costs for the licensed products until December 31, 2017. Thereafter, we will be responsible for 55.0% of the budgeted research and development costs related to the licensed products in the licensed territory, and Sarepta will be responsible for 45.0% of such costs. Any costs in excess of 110.0% of the budgeted amount are borne by the party that incurred such costs. We are also obligated to spend a specified minimum amount on the research and development of certain licensed products prior to the end of 2019.

Manufacture and Supply of Licensed Products

We have agreed to use commercially reasonable efforts to supply to Sarepta active pharmaceutical ingredient, finished drug product and placebo for Sarepta to conduct research, development and commercialization activities for the licensed products in accordance with the license and collaboration agreement. Sarepta also will have the right to establish back up and second source suppliers under certain circumstances.

Intellectual Property

Under the terms of the license and collaboration agreement, each party will own the entire right, title and interest in and to all know-how and patent rights first made or invented solely by the employees or consultants of such party in the course of the collaboration, and all such know-how and patent rights will be included in the licenses granted to the other party under the license and collaboration agreement. The parties will jointly own all rights, title and interests in and to all know-how and patent rights first made or invented jointly by employees or consultants of the parties in the course of the collaboration.

Latin America Option

Under the license and collaboration agreement, Sarepta has an exclusive option, which we refer to as the Latin America Option to expand the licensed territory to include specified countries in South and Central America, which we refer to as the Option Territory. Sarepta may exercise the Latin America Option at any time prior to the date that is three months following the first receipt of regulatory approval for a licensed product in the United States or the European Union. We are eligible to receive from Sarepta up to an aggregate of

$17.0 million for the exercise of the Latin America Option and the achievement of certain regulatory milestones. If Sarepta exercises the Latin America Option, it will be solely responsible for all research, development and commercialization costs of the licensed products that are specific to the Option Territory. We are also be eligible to receive up to $82.5 million in specified sales milestones on a product-by-product basis in the Option Territory, as well as royalties at the same rates as elsewhere in the licensed territory.

Commercialization

Under the license and collaboration agreement, Sarepta will be solely responsible for all commercialization activities and associated costs, relating to licensed products in the licensed territories. Sarepta has agreed to use commercially reasonable efforts to commercialize licensed products in specified countries within the licensed territories and, if the Latin America Option is exercised, to use commercially reasonable efforts to commercialize licensed products in certain specified countries within the Option Territory.

Termination Provision

Unless earlier terminated, the license and collaboration agreement will expire on a licensed product-by-licensed product and country-by-country basis upon the expiration of the royalty term in such country for such licensed product. The license and collaboration agreement may be terminated by Sarepta upon six months' prior written notice in its entirety or on a licensed product-by-licensed product and country-by-country basis. Either party may, subject to a cure period, terminate the license and collaboration agreement in the event of the other party's uncured material breach. Sarepta may also terminate the license and collaboration agreement under specified circumstances relating to the safety or regulatory approvability of ezutromid. Except with respect to a second generation or future generation small molecule utrophin modulator candidate that the joint steering committee elects not to pursue, as described above, during the term of the license and collaboration agreement the parties are prohibited from commercializing small molecule utrophin modulators anywhere in the world outside of the collaboration. Such exclusivity commitment may survive for one year following termination with respect to one party depending upon the circumstances of termination.

Standstill Provision

The license and collaboration agreement also contains a standstill provision pursuant to which, among other things, each party has agreed that, for a period from the execution of the license and collaboration agreement until the date that regulatory approval is first received for a licensed product, subject to certain exceptions, or unless invited in writing by the other party to do so, neither party nor its respective affiliates will, directly or indirectly: (i) effect or seek, offer or propose to effect, or cause or participate in any acquisition of securities or assets of the other party; any tender or exchange offer, merger, consolidation or other business combination involving the other party; any recapitalization, restructuring, liquidation, dissolution or other extraordinary transaction with respect to the other party; or any "solicitation" of "proxies" or consents to vote any voting securities of the other party, or in any way advise or, assist any other person in doing so; (ii) form, join or in any way participate in a "group" with respect to any securities of the other party; (iii) act in concert with any person in relation to voting securities of the other party; (iv) otherwise act to seek to control or influence the management, board of directors or policies of the other party; (v) take any action reasonably expected to force the other party to make a public announcement regarding any such matters; or (iv) enter into any agreements, discussions or arrangements with any third party with respect to any of the foregoing.

University of Oxford

In November 2013, we acquired all of the outstanding equity of MuOx Limited, or MuOx, a spin out of the University of Oxford founded by Professors Stephen Davies and Kay Davies. MuOx is our wholly owned subsidiary. In connection with that acquisition, we and MuOx entered into a set of agreements with the University of Oxford and its technology transfer division, Isis Innovation Limited, or Isis, which is now known

as Oxford University Innovation Limited, regarding the development of small molecule utrophin modulators. In November 2015, this set of agreements were extended through November 2019, with an option to extend for a further twelve months.

Research Sponsorship

We have agreed to fund a drug research and discovery program in the University of Oxford laboratories to identify and research utrophin modulators to treat DMD. The University of Oxford is responsible for conducting this program. Isis has no obligations under the research sponsorship agreement. We refer to the agreement that governs our research sponsorship with the University of Oxford, which we, the University of Oxford and Isis entered into in November 2013, amended and restated in July 2014 and amended in November 2015, as the research sponsorship agreement. Under the research sponsorship agreement, we have agreed to fund up to £4.3 million over a six-year research period ending in November 2019. If we exercise our right to extend the research period by an additional year, we have agreed to fund an additional £0.8 million, for a total of £5.2 million. As of January 31, 2017, we had paid the University of Oxford £2.1 million of this amount.

The University of Oxford will own all intellectual property arising from the sponsored research, and we have agreed to assign to the University of Oxford any intellectual property arising from the sponsored research that either we or third parties whom we engage, may create, subject to our exercise of an option to obtain an exclusive license under the intellectual property arising from the sponsored research, as described below.

Either we or the University of Oxford would have the right to terminate the research sponsorship agreement for specified reasons, including the other party's insolvency or material breach, if the breach remains uncured for a specified period or is uncurable, or our mutual determination, at specified times, that there are valid scientific reasons for terminating the project. The University of Oxford may also terminate the research sponsorship agreement if we default on more than one payment obligation and do not remedy the failure within a specified period after receiving notice. We may also terminate the research sponsorship agreement, after a specified period of time if any of the principal investigators is unable or unwilling to continue supervising the sponsored research and the successor proposed by the University of Oxford is not acceptable to us on reasonable and substantial grounds.

License of Know-How

In November 2013, Isis executed a know-how license agreement with MuOx. We refer to the agreement, which was amended in July 2014 and March 2017, as the know-how license agreement. In the know-how license agreement, Isis granted MuOx a license under specified know-how, consisting of data and other information associated with specified utrophin modulators and biological screening technology, and all intellectual property rights pertaining to the specified know-how, to research, develop, make, have made, use, have used, import, have imported, export, have exported, and market the licensed know-how and products or processes resulting from the licensed know-how. The know-how license agreement was novated to us in March 2017. We refer to the know-how specified in the know-how license agreement, as Oxford's background know-how. Our rights under Oxford's background know-how in the specified utrophin modulators are exclusive and sublicenseable. Our rights under Oxford's background know-how in the biological screening technology were initially exclusive, but became non-exclusive in November 2016. In March 2017, we amended the know-how license agreement to extend our rights under Oxford's background know-how for certain biological screening technologies to November 2019, subject to an option to extend for a further twelve months. We paid to Isis a nominal amount upon its entry into the amendment in March 2017 and, pursuant to the amendment, agreed to pay to Isis additional nominal amounts annually for the term of the exclusive license period, provided the amounts payable in the final year of the exclusive license period shall only be payable if we further extend the term of the exclusive license. Our rights under the know-how license agreement are sublicenseable with Isis' consent, which may not be unreasonably withheld. Our rights are also subject to the rights of the University of Oxford, the Muscular Dystrophy Association and the Muscular Dystrophy Campaign, and their respective employees,

students and agents, to use and publish Oxford's background know-how for specified scholarly and academic research and teaching purposes. We have agreed to use commercially reasonable efforts to develop, exploit and market Oxford's background know-how or any compound deriving from Oxford's background know-how.

The know-how license agreement will remain in effect at least until November 2019 with respect to the biological screening technology know-how, and otherwise, with respect to each of the compound or biological screening technology know-how, as long as we or our sublicensees are using commercially reasonable efforts to research and develop compounds derived from that know-how. Either we or Isis would have the right to terminate the know-how license agreement if the other party materially breaches the know-how license agreement and the breach remains uncured for a specified period or is uncurable. We may terminate the know-how license agreement at our discretion by giving Isis six months' prior written notice. Isis may terminate the know-how license agreement on thirty days' notice if we fail to use commercially reasonable efforts to exploit Oxford's background know-how and do not remedy such breach within a specified time, or immediately, if we take specified actions relating to winding up or experience certain insolvency-related events.

Exclusive Option Rights

We refer to the intellectual property rights arising under the research sponsorship agreement, or arising from research and development of small molecule utrophin modulation conducted by or under the supervision of certain University of Oxford scientists, that is created or reduced to practice after November 2013 and within a specified time after the expiration or termination of the research sponsorship agreement, as arising IP. Under an option agreement that we, the University of Oxford and Isis entered into in November 2013 and amended in November 2015, which we refer to, as amended, as the option agreement, Isis granted us an exclusive option to license the arising IP. We paid Isis £10,000 in connection with entering into the amendment to the option agreement. We may exercise the option within specified periods.

In connection with entering into the initial option agreement, we paid Isis an option fee of a specified amount and issued to Isis warrants to purchase up to 354,090 of our ordinary shares at a purchase price of £0.20 per ordinary share. In connection with the November 2015 amendment, we extended the period during which Isis may exercise such options to February 2020. The warrants may be exercised based on the achievement of certain research, development and regulatory milestones. In November 2015, we announced the nomination of two series of new utrophin modulators for progression into lead optimization studies to achieve the first research milestone. This entitled Isis to subscribe for 50,000 new ordinary one penny shares at an exercise price of 20 pence per share during the three month period starting November 22, 2016, all of which were exercised in February 2017.

If we exercise our option to obtain a license under arising IP, we would be obligated to pay Isis up to a specified sum in option exercise fees, and Isis will use reasonable efforts to enter into a license agreement as quickly as possible, subject to Isis obtaining all necessary intellectual property assignments and conducting its internal due diligence procedures.

For any arising IP for which we have exercised the option and that comprises new chemical entities or compounds, which we refer to as optioned compounds, we would obtain an exclusive, sublicenseable license. We are obligated to pay milestone payments of up to £75,000 upon the achievement of specified development milestones, whether such milestones occur prior to or after our exercise of the option to obtain an exclusive sublicenseable license. Following exercise of such an option we would also be obligated to pay milestone payments upon the achievement of specified regulatory milestones with respect to each optioned compound. The specified regulatory milestone payment is due each time the specified regulatory milestone is achieved with respect to an optioned compound and, if each optioned compound achieved each regulatory milestone once, we would be obligated to pay Isis a total of £3.7 million in regulatory milestone payments for each optioned compound.

We would also be obligated to pay Isis a low single digit royalty of net sales by us, our affiliates or sublicensees of any product containing an optioned compound, which we refer to as a licensed product, subject to specified

reductions. Our obligation to pay the royalty would expire on the later of the expiration of the last valid claim of any licensed patent or patent application claiming the licensed product or 20 years after the date on which we enter the license agreement. We would also be obligated to pay Isis a low single digit percentage of any payments we receive in connection with granting a sublicense under the licensed arising IP.

If we funded the development of the arising IP for the optioned compounds, through our funding under the research sponsorship agreement or by funding work at contract research organizations prior to the creation of the arising IP, then the milestone and royalty payments will be reduced to reflect the value that our funding delivered to the arising IP. We and Isis would negotiate such adjustment in good faith. If we and Isis are unable to agree, an expert will be appointed to make the determination.

For any arising IP for which we have exercised the option and that does not comprise new chemical entities or compound, which we call enabling IP, we would obtain an exclusive license, which we could sublicense with Isis' prior written consent, not to be unreasonably withheld, delayed or conditioned. We and Isis would negotiate the milestone payments and any other payments that we would be obligated to pay to Isis with respect to enabling IP. If we and Isis are unable to agree, an expert will be appointed to make the determination.

Any license granted to us under arising IP would be subject to the rights of the University of Oxford, and any person who at any time worked on the licensed arising IP, to use and publish the arising IP for specified scholarly and academic research and teaching purposes. We would also be obligated to use commercially reasonable efforts to develop, exploit and market the arising IP licensed to us.

The license agreement would remain in effect as long as we are using commercially reasonable efforts to develop and market the licensed products, unless terminated earlier by us or Isis, or extended by mutual agreement. Either we or Isis would be permitted to terminate the license agreement at any time if the other party materially breaches the license agreement and the breach remains uncured for a specified period or the breach is uncurable. We would be permitted to terminate the license agreement for any reason after it has been in effect for three years upon giving six months' prior written notice. Isis would be permitted to terminate the license agreement if we challenge the validity of the licensed patents or patent applications or if we claim that we are no longer obligated to make payments to Isis under the license agreement because the know-how is unnecessary, or if we take specified actions relating to winding up or experience certain insolvency-related events. Upon termination of the license agreement, we would be obligated to grant Isis an irrevocable, transferable, non-exclusive license to develop, make, have made, use and market any improvements made by us during the option period, and related intellectual property rights, subject to the payment of a reasonable royalty.

The option agreement will remain in effect until a specified period of time, sufficient for us and Isis to enter into the license agreement, after our rights to exercise the options terminate, unless the option agreement is terminated earlier by either Isis and the University of Oxford, or us. Either we, or Isis and the University of Oxford, may terminate the option agreement at any time if the other materially breaches the option agreement and the breach remains uncured for a specified period or the breach is uncurable, or if the other becomes insolvent. We may also terminate the option agreement, effective on each anniversary of the effective date of the option agreement, by giving 60 days' prior written notice to Isis and the University of Oxford.

Wellcome Trust

In October 2012, we entered into a translation award funding agreement with the Wellcome Trust Limited, as trustee of the Wellcome Trust, in order to support a Phase 1 and a Phase 2 clinical trial of ridinilazole for the treatment of CDI. We refer to the translation award funding agreement as the translation award agreement. Under the translation award agreement, we were eligible to receive up to £4.0 million from the Wellcome Trust, of which we have received the entire £4.0 million. The translation award agreement followed a funding agreement we and the Wellcome Trust entered in October 2009, which we refer to as the discovery award agreement, under which we received £2.3 million for preclinical development of CDI antibiotics. We refer to any compound or

product that is covered by intellectual property rights created under the discovery award agreement or the translation award agreement, or that is covered by intellectual property rights that we created or to which we had rights prior to October 2009 and that relate to the activities under the discovery award agreement or the translation award agreement, as the award products. We have agreed to use commercially reasonable efforts to achieve certain development milestones by specified dates.

Development and Commercialization Obligations

Under the translation award agreement, we may, subject to the Wellcome Trust's prior written consent, which will not be unreasonably withheld or delayed, conduct the development and commercialization of award products. We may conduct these activities, which we refer to as exploitation, ourselves or through our affiliates, licensees and third-party collaborators. We refer to any intellectual property rights associated with the exploitation as exploitation IP. The Wellcome Trust's consent is contingent on the establishment of a revenue sharing agreement that incorporates the financial terms of the translation award agreement. We are required to ensure that any results generated by a third party with whom we collaborate or subcontract will be deemed exploitation IP.

If we or our licensees do not develop or commercialize any exploitation IP in specified markets or specified indications within specified timeframes, the Wellcome Trust is permitted to conduct exploitation of the exploitation IP in those markets or indications. If the Wellcome Trust exercises its exploitation right, we would license or assign to it the appropriate exploitation IP and grant it non-exclusive, royalty-free licenses to our related background intellectual property, and we would be entitled to receive a portion of the net revenue received by the Wellcome Trust from exercise of its exploitation rights.

We may not allow a lien to be imposed on the exploitation IP or on any intellectual property rights licensed to us that we used for the clinical trials funded by the Wellcome Trust, except for certain liens arising in the ordinary course of business. We may also not make any material change to the general nature of our business without consent from the Wellcome Trust.

Financial Terms

Under the terms of the translation award agreement and the terms of the revenue sharing agreement we would enter into with the Wellcome Trust to permit our exploitation of the exploitation IP or award products, the Wellcome Trust is entitled to a share of the cumulative net revenue that we, our affiliates, licensees or third party collaborators receive from exploiting the exploitation IP or award products. The Wellcome Trust would be eligible to receive a tiered portion of the net revenue, ranging from a mid-single digit percentage up to a mid-twenties percentage. Under the translation award agreement, if we, the Wellcome Trust or a third party contributes funding to further develop the exploitation IP or award products, we and the Wellcome Trust will negotiate in good faith modifications to the net revenue sharing percentage to reflect changes in our respective proportionate development costs for award products. If we and the Wellcome Trust are unable to agree, an expert will be appointed to make the determination. We also agreed not to accept funding to complete the Phase 2 clinical trial, without the Wellcome Trust's consent, if such funding would materially prejudice the Wellcome Trust's net revenue sharing or exploitation rights. In addition, we would be obligated to pay the Wellcome Trust a milestone in a specified amount if cumulative net revenue exceeds a specified amount.

The revenue sharing agreement would last until the latest of the expiration of the last patent or patent application covering any invention arising out of our activities under the research and clinical trials funded by the Wellcome Trust or the expiration of any agreement or payment obligations relating to exploitation of the intellectual property rights arising out of our activities under the research and clinical trials funded by the Wellcome Trust under the translation award agreement. Either we or the Wellcome Trust would have the right to terminate the revenue sharing agreement if the other party materially breaches the revenue sharing agreement and the breach remains uncured for a specified period or the breach is uncurable, if the other party experiences specified insolvency related events, or if the translation award agreement expires or is terminated.

If we are obligated to withhold tax on the amounts payable to the Wellcome Trust, we agree to pay the Wellcome Trust a greater amount, such that the Wellcome Trust receives the same amount after the withholding as it would have received without such deduction.

We would be required to make a full or partial repayment to the Wellcome Trust of the funding we received under the translation award agreement, plus accrued interest, under specified conditions, including our unauthorized use of the award amount, our fraudulent or willful misconduct, our knowingly withholding material information from the Wellcome Trust, or an acquisition by certain third parties of all or a material part of our business or assets or of a majority of our equity. Upon such a full repayment, our obligation to share a portion of net revenue with the Wellcome Trust would terminate.

Termination

Unless earlier terminated by the Wellcome Trust, the translation award agreement will terminate on the earlier of our full repayment of the award amount, plus accrued interest, to the Wellcome Trust following its request for repayment, or the expiration of all payment obligations under the translation award agreement and the revenue sharing agreement. The Wellcome Trust may terminate the translation award agreement for specified reasons, including our material breach or insolvency related events or the Wellcome Trust's determination that the clinical trials should be terminated due to a serious failure in the progress, management or conduct of the clinical trials, if we do not remedy such condition within a specified period after receiving notice.

Assignment

We may not, without the Wellcome Trust's prior consent, assign, transfer or declare a trust over the translation award agreement or otherwise dispose of any of our rights or obligations under the translation award agreement, with such consent not being unreasonably withheld, delayed or conditioned, other than an assignment to our affiliates.

Muscular Dystrophy Association

In December 2011, we entered into a grant agreement with the Muscular Dystrophy Association, Inc., or MDA, a not for profit corporation based in New York, in order to partially fund a Phase 1 clinical trial of ezutromid to treat DMD. We refer to this grant agreement with MDA as the MDA grant agreement. To date, we have received the entire amount of MDA's grant to us, or an aggregate of $750,000.

Financial Terms

We refer to small molecules that can upregulate the utrophin gene, including ezutromid and compounds with similar mechanisms of action to which we have rights, as project compounds. Under the MDA grant agreement, we have agreed to make specified milestone payments to MDA during our or our affiliates' development and commercialization of pharmaceutical products containing the project compounds, which we refer to as project products. Because we raised more than a specified aggregate amount of funding, we have paid a specified sum to MDA under the terms of the agreement, which we refer to as the MDA cash infusion milestone payment.

We have also agreed to pay MDA a specified lump sum amount, less any previously paid MDA cash infusion milestone payment, following the regulatory approval of any project product for use or sale in the United States or European Union for the treatment of DMD or Becker muscular dystrophy, or BMD, and an additional specified sum upon achievement of a commercial milestone. We would be obligated to pay MDA a low single digit percentage royalty of worldwide net sales by us, our affiliates or licensees of any project product. If we assign our rights to any of the project compounds or experience specified change in control events, MDA may require our assignee to assume our obligations under the MDA grant agreement with respect to the assigned rights, or require us to pay MDA the greater of a low single digit percentage of the fair market value of the assigned rights, or an amount that would give MDA an internal rate of return of a low double digit percentage on its grant to us.

Interruption License

Upon the occurrence of specified events, which we refer to as interruptions, we have agreed to refund to MDA the entire grant amount of $750,000 plus a low double digit interest on that amount, subject to specified exemptions. An interruption may occur if we or our affiliates cease reasonable research, development and commercialization of project products and cease using diligent efforts to obtain a third party development and commercialization partner, which require our annual expenditure of a minimum specified amount on such efforts for longer than a specified period. Interruptions may also occur if we license the rights to develop and commercialize project products to a third party without retaining a right of reversion, and such partner ceases reasonable development and commercialization of project products for longer than a specified period or ceases to sell project products in the United States or European Union, or if we, upon the reversion of such rights from a third party commercialization partner to us, fail to use reasonable efforts to develop and commercialize project products and cease using diligent efforts to obtain a third party development and commercialization partner, or, within a specified period from the date of reversion, to license the development and commercialization activities of project products to a third party. In all such cases, we are exempt from interruption payments in the event of specified scientific failures, including if we fail to achieve primary endpoints for any clinical trial of ezutromid, if the project compounds are unfit for administration to humans or if we cannot develop a commercial manufacturing process.

We have granted to MDA, effective on the occurrence of such an interruption, an exclusive, sublicenseable, worldwide, perpetual, irrevocable and royalty-free license under the patent rights, know-how and intellectual property that we control, useful for the project compounds or project products, to research, develop, manufacture, have manufactured, use, sell, offer to sell, import and export the project compounds and project products for the prevention, treatment, or amelioration of DMD or BMD. We refer to such license as the interruption license. Upon the effectiveness of the interruption license, we would be obligated to assign to MDA or its designee the regulatory filings, regulatory approvals, and contract rights that we or our affiliates own, and deliver specified know-how, in each case, relating to project compounds and project products.

MDA acknowledges that if a royalty or other payment is due to any third party from whom we licensed or acquired the intellectual property licensed to MDA, the interruption license is contingent on MDA or its sublicensee assuming those obligations resulting from their exercise of the interruption license.

Termination

The MDA grant agreement will continue indefinitely.

Duchenne Partners Fund

In December 2011, we entered into a grant agreement with the Duchenne Partners Fund, LLC, or DPF, a Delaware limited liability company, in order to partially fund a Phase 1 clinical trial of ezutromid to treat DMD. We refer to this grant agreement with DPF as the DPF grant agreement. To date, we have received the entire amount of DPF's grant to us, or an aggregate of $500,000.

Financial Terms

We refer to small molecules that can upregulate the utrophin gene, including ezutromid and compounds with similar mechanisms of action to which we have rights, as project compounds. Under the DPF grant agreement, we have agreed to make specified milestone payments to DPF during our or our affiliates' development and commercialization of pharmaceutical products containing the project compounds, which we refer to as project products. Because we raised more than a specified aggregate amount of funding, we have paid a specified sum to DPF under the terms of the agreement, which we refer to as the DPF cash infusion milestone payment.

We have also agreed to pay DPF a specified lump sum amount, less any previously paid DPF cash infusion milestone payment, following the regulatory approval of any project product for use or sale in the United States or European Union for the treatment of DMD or BMD and an additional specified sum upon achievement of a commercial milestone. We would be obligated to pay DPF a low single digit percentage royalty of worldwide net sales by us, our affiliates or licensees of any project product. If we assign our rights to any of the project compounds or experience specified change in control events, DPF may require our assignee to assume our obligations under the DPF grant agreement with respect to the assigned rights, or require us to pay DPF the greater of a low single digit percentage of the fair market value of the assigned rights, or an amount that would give DPF an internal rate of return of a low double digit percentage on its grant to us.

Termination

The DPF grant agreement will continue indefinitely.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technologies, knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions, government agencies and private and public research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain marketing approvals for their products more rapidly than we obtain approval for ours. In addition, our ability to compete may be affected because in some cases insurers or other third-party payors seek to encourage the use of generic products. This may have the effect of making branded products less attractive, from a cost perspective, to buyers.

The key competitive factors affecting the success of our product candidates are likely to be their efficacy, safety, convenience, price and the availability of coverage and reimbursement from government and other third-party payors.

The competition for ezutromid and ridinilazole includes the following:

Ezutromid

There is currently no approved therapy for the treatment of DMD applicable to all DMD patients that seeks to alter the progression of the disease. Corticosteroids, such as prednisolone and deflazacort, are the current standard of care for DMD patients, although these are symptomatic treatments that do not address the underlying cause of DMD, and their use can be associated with severe side effects and concerns over weight gain. Other companies are developing alternative therapeutic approaches to the treatment of DMD, a number of which are outlined below.

Exon Skipping. Sarepta and BioMarin Pharmaceutical Inc., or BioMarin, following the acquisition of Prosensa Holding N.V. in 2015, are developing treatments for DMD based on exon-skipping approaches. Exons are organic molecules known as nucleotides within the DNA strand that the cellular machinery translates to make full-length, functional protein. In a sub-population of DMD patients, synthesis of the dystrophin protein is disrupted because of mutations that may be due, among other things, to deleted exons. Exon-skipping technology seeks to allow the production of a shorter but still functional dystrophin protein. Sarepta is developing treatments for DMD based on exon-skipping approaches and received accelerated approval from the FDA for eteplirsen in September 2016. Eteplirsen targets exon 51 and targets approximately 13% of all DMD patients. Sarepta is also developing other exon-skipping therapies to treat other genetic mutations. Sarepta also has product candidates in clinical trials that are targeting exon 44, which is applicable to approximately 6% of all DMD patients, exon 45, which is applicable to approximately 8% of all DMD patients, and exon 53, which is applicable to approximately 6% of all DMD patients. According to an article published in 2009 in the peer reviewed journal *Human Mutation*, skipping of the ten most common exons would treat in the aggregate approximately 41% of all DMD patients. We believe that there are exon-skipping therapies currently in clinical development to address four of these exons and that skipping of these exons would treat in the aggregate less than one-third of all DMD patients. BioMarin's most advanced exon-skipping drug is drisapersen (Kyndrisa™). BioMarin completed a rolling NDA submission for drisapersen to the FDA seeking accelerated approval in April 2015. In January 2016, the FDA issued to BioMarin a "complete response" letter to the NDA for drisapersen which indicated that the review cycle for the application was complete and that the application was not ready for approval in its present form due to the standard of substantial evidence of effectiveness having not been met. In May 2016, BioMarin announced it had withdrawn its market authorization application, or MAA, to the EMA for drisapersen, and that it was discontinuing development of drisapersen and three other exon-skipping therapies in clinical trials that targeted exon 44, exon 45 and exon 53, respectively. BioMarin stated it would continue to explore the development next generation exon-skipping therapies. Other companies developing therapies targeting exon mutations include Nippon Shinyaku Co., Ltd., which is conducting Phase 2 clinical trials in Japan and the United States and Wave Life Sciences Ltd., which expects to commence clinical trials by mid-2017.

Nonsense mutations. PTC Therapeutics, Inc., or PTC, is developing ataluren (Translarna™). Ataluren is a small molecule that enables formation of functional dystrophin in DMD patients with nonsense mutations. DMD caused by nonsense mutations affects approximately 13% of all DMD patients. The European Commission has granted conditional approval for ataluren in Europe, and PTC is commercializing ataluren in several European countries. In October 2015, PTC announced a Phase 3 confirmatory clinical trial of ataluren did not achieve its primary endpoint, and in February 2016, PTC announced its receipt of a "refuse to file" letter from the FDA indicating that PTC's NDA for ataluren was not sufficiently complete to permit a substantive review. In March 2017, PTC announced the filing over protest of the NDA for ataluren, which allows PTC to have its NDA filed and reviewed following receipt of a refuse to file determination. The FDA granted a Prescription Drug User Fee Act, or PDUFA, date for ataluren of October 24, 2017.

Other DMD approaches. A number of other companies are pursuing alternative therapeutic approaches for the treatment of DMD. Tivorsan Pharmaceuticals is developing a recombinant form of biglycan, a protein that is naturally produced in the body and regulates production of utrophin in developing muscle, which is currently in preclinical development. Pfizer, Inc., or Pfizer, and Bristol-Myers Squibb Company, or BMS, are pursuing an approach based on muscle tissue growth through myostatin inhibition. Pfizer is developing the myostatin inhibitor PF-06252616 (domagrozumab) and initiated a Phase 2 clinical trial in patients with DMD in December 2014. BMS is developing BMS-986089, a myostatin inhibitor that is currently in Phase 1/2 clinical development. Santhera Pharmaceuticals Holding AG, or Santhera, completed a Phase 3 clinical trial of its product candidate, idebenone (Raxone®/Catena®), in 2014 and reported that idebenone delayed deterioration in respiratory function. Santhera filed a MAA to the EMA in 2016. Santhera is conducting a confirmatory Phase 3 trial of idebenone in DMD patients who are receiving concomitant corticosteroids. Catabasis Pharmaceuticals, Inc., or Catabasis, is developing edasalonexent as a non-steroidal, anti-inflammatory drug. Catabasis reported top-line results in January 2017 from a Phase 1/2 clinical trial in which edasalonexent did not meet its primary endpoint. Catabasis expects to report results from an open-label extension of this Phase 1/2 clinical trial in 2017. Akashi

Therapeutics, or Akashi, is developing HT-100, an anti-inflammatory and anti-fibrotic small molecule that aims to reduce fibrosis and inflammation. In February 2016, Akashi announced that dosing and enrollment into a Phase 1b/2a clinical trial of HT-100 was suspended due to a fatality in the trial. A number of companies are targeting gene therapy based approaches for DMD which has the potential to address the genetic cause of DMD by using an adeno-associated virus to deliver a shortened, yet functional, version of the dystrophin gene to a DMD patient. Companies with gene therapy based approaches include Asklepios Biopharmaceuticals, Inc., which is developing biostrophin and is currently in Phase 1 clinical development, and Solid Biosciences, which expect to commence clinical trials in patients with DMD in 2017.

Ridinilazole

Several pharmaceutical and biotechnology companies have established themselves in the market for the treatment of CDI, and several additional companies are developing products for the treatment of CDI. We expect that these products will compete with ridinilazole.

Antibiotics. The current standard of care for CDI is treatment with the broad spectrum antibiotics vancomycin and metronidazole, both of which are available in generic form in the United States. Generic antibiotic therapies typically are sold at lower prices than branded antibiotics and generally are preferred by managed care providers of health services. The antibiotic fidaxomicin (Dificid™ in the United States, Dificlir™ in Europe) is approved for the treatment of CDI in the United States and the European Union. Fidaxomicin was originally developed by Optimer Pharmaceuticals, Inc., which was later acquired by Cubist Pharmaceuticals, Inc., or Cubist. Cubist was recently acquired by Merck & Co., Inc., or Merck. Antibiotics in late-stage clinical trials for treatment of CDI include cadazolid, which was originally being developed by Actelion Pharmaceuticals Limited, or Actelion. Johnson and Johnson acquired global rights to cadazolid as part of the acquisition of Actelion announced in January 2017. Cadazolid is currently in Phase 3 clinical development.

Other CDI approaches. A number of other approaches for the treatment of CDI are in development. Merck is developing the monoclonal antibodies bezlotoxumab (Zinplava™), which received approval from the FDA in October 2016. Bezlotoxumab is an antibody that neutralizes certain toxins that are produced by *C. difficile* bacteria and indicated to reduce recurrence for CDI in patients who are receiving antibacterial drug treatment and are at high risk of disease recurrence. Merck has also filed a MAA with the EMA, and the CHMP of the EMA issued an opinion recommending approval of bezlotoxumab in November 2016. Sanofi Pasteur SA is developing the vaccine ACAM-CDIFF for primary prevention of CDI. ACAM-CDIFF is likely to be used only in high-risk patients given the difficulty of administering a vaccine to a broad population. Pfizer is developing the vaccine PF-06425090 that aims to induce a functional antibody response to neutralize the *C difficile* bacterial toxins. Pfizer reported top-line Phase 2 results in January 2017 and expects to progress into Phase 3 trial in the first half of 2017.

Fecal biotherapy aims to recolonize the bacteria that comprise the natural gut flora and would also be adjunctive therapy to antibiotics. Fecal biotherapy approaches in development include SER-109 and SER-262, which are being developed by Seres Therapeutics Inc., formerly Seres Health, Inc., and RBX2660, which is being developed by Rebiotix Inc. Seres reported interim results from a Phase 2 trial in 2016 in which SER-109 missed its primary efficacy endpoint while SER-262 is currently being evaluated in a Phase 1b clinical trial in CDI patients. RBX2660 is currently being evaluated in a Phase 2 clinical trial. Synthetic Biologics, Inc., is developing ribaxamase, an oral enzyme designed to degrade certain IV beta-lactam antibiotics within the GI tract to preserve the natural balance of the microbiome and reduce the risk of colonization by bacterial including *C. diffiicle*. In January 2017, it was reported that ribaxamase met its primary endpoint in a Phase 2b clinical trial.

Manufacturing

We do not own or operate, and currently have no plans to establish, manufacturing facilities for the production of clinical or commercial quantities of ezutromid, ridinilazole or for the other compounds that we are evaluating in

our DMD program. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates and any products that we may develop.

We currently engage a single, third-party manufacturer to provide clinical material of the active pharmaceutical ingredient, or API, and fill and finish services for the final drug product of the F3 formulation of ezutromid that is being used in our Phase 2 proof of concept clinical trial. We are engaged with a different drug product manufacturer to provide bulk drug product of the F6 formulation of ezutromid. A different third party manufacturer provides fill and finish services to supply the final drug product of the F6 formulation of ezutromid that is being evaluated in our ongoing Phase 2 proof of concept clinical trial. We are engaged with a different third-party vendor to provide labelling, packaging and distributions services of the F3 and F6 formulations. We are engaged with another third-party manufacturer to provide clinical material of the API of ridinilazole with a different supplier responsible for fill and finish services to supply the final drug product for use in the future Phase 3 clinical trials. We believe these suppliers are suitable for commercial manufacture. We expect to use the same third-party vendor for clinical packaging, labelling and distribution of the finalized ridinilazole drug product that we use for ezutromid. We obtain the supplies of our API and drug products from these manufacturers pursuant to agreements that include specific supply timelines and volume expectations.

We obtain the supplies of our product candidates from these manufacturers under master services contracts and specific work orders. However, we do not have long-term supply arrangements in place. We do not currently have arrangements in place for redundant supply or a second source for API for ezutromid. If any of our current manufacturers should become unavailable to us for any reason, we believe that there are a number of potential replacements, although we might incur some delay in identifying and qualifying such replacements.

All of our product candidates are organic compounds of low molecular weight and are referred to as small molecules. We have selected these compounds based on their potential efficacy and safety, although they are also associated with reasonable cost of goods, ready availability of starting materials and ease of synthesis. We believe that the chemistry for ezutromid and ridinilazole is amenable to scale-up and does not currently require unusual equipment in the manufacturing process. We expect to continue to develop product candidates that can be produced cost-effectively at contract manufacturing facilities.

Intellectual Property

Our success depends in large part on our ability to obtain and maintain proprietary protection for our product candidates, technology and know-how, to operate without infringing the proprietary rights of others and to prevent others from infringing our proprietary rights. We strive to protect the proprietary technology that we believe is important to our business by, among other methods, seeking and maintaining patents, where available, that are intended to cover our product candidates, compositions and formulations, their methods of use and processes for their manufacture and any other inventions that are commercially important to the development of our business. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary and competitive position.

As of March 15, 2017, we owned or exclusively licensed a total of eight U.S. patents, three U.S. patent applications, six European patents and three European patent applications, including original filings, continuations and divisional applications, as well as numerous other foreign counterparts to these U.S. and European patents and patent applications.

Our DMD patent portfolio includes the following granted patents and patent applications that we own or exclusively license:

- two granted U.S. patents covering the composition of matter of ezutromid and combinations of ezutromid with ancillary therapeutic agents, which are scheduled to expire in 2029 and 2030, respectively;

- a granted U.S. patent covering methods of manufacture of ezutromid, which is scheduled to expire in 2029;
- a granted U.S. patent covering formulations of ezutromid, which is scheduled to expire in 2033;
- a granted European patent covering the composition of matter of ezutromid that cleared the opposition period in April 2015 with no opposition filed against it at the European Patent Office, and which is scheduled to expire in 2027;
- a granted European patent covering formulations of ezutromid, which is scheduled to expire in 2033;
- a granted European patent covering combinations of ezutromid with ancillary therapeutic agents, which is scheduled to expire in 2028; and
- a number of pending patent applications covering formulations of ezutromid, further methods of use of ezutromid and the composition of matter of second generation utrophin modulator candidates.

Our CDI patent portfolio includes the following granted patents and patent applications that we own or exclusively license:

- a granted U.S. patent covering the use of ridinilazole in the treatment of CDI, which is scheduled to expire in 2029;
- a corresponding granted European patent covering the use of ridinilazole in the treatment of CDI, which is scheduled to expire in 2029;
- a granted U.S. patent covering hydrates of ridinilazole, which is scheduled to expire in 2029;
- a granted European divisional patent covering hydrates of ridinilazole and pharmaceutical compositions comprising ridinilazole;
- a further pending U.S. continuation patent application;
- a further pending divisional application with the European Patent Office; and
- two granted U.S. patents, a pending U.S. continuation patent application, a granted European patent and a pending European divisional application covering second generation agents for the treatment of CDI, which are scheduled to expire in 2031.

While patent protection is not available for composition of matter claims that only recite the API for ridinilazole, protection may be available for the pharmaceutical compositions comprising ridinilazole as well as other forms thereof such as hydrates (and indeed claims have been secured for the latter in both Europe and theUnited States).

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the filing date of a non-provisional patent application. In the United States, a patent's term may, in certain cases, be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office, or the USPTO, in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent.

The term of a U.S. patent that covers a drug, biological product or medical device approved pursuant to a pre-market approval, or PMA, may also be eligible for patent term extension when FDA approval is granted, provided that certain statutory and regulatory requirements are met. The length of the patent term extension is related to the length of time the drug is under regulatory review while the patent is in force. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration date set for the patent. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be granted an extension and only those claims reading on the

approved drug may be extended. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug, provided that statutory and regulatory requirements are met. Thus, in the future, if and when our product candidates receive approval by the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents covering those products, depending upon the length of the clinical trials for each drug and other factors. The expiration dates of our patents and patent applications referred to above are without regard to potential patent term extension or other market exclusivity that may be available to us.

In addition to patents, we may rely, in some circumstances, on trade secrets to protect our technology and maintain our competitive position. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by confidentiality agreements with our employees, corporate and scientific collaborators, consultants, scientific advisors, contractors and other third parties. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems.

Sales and Marketing

In light of our stage of development, we have not yet established a sales and marketing organization or distribution capabilities. Under the terms of our exclusive license and collaboration agreement with Sarepta, we have granted Sarepta the exclusive right to commercialize products in our utrophin modulator pipeline, including our lead candidate ezutromid, in the European Union, Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States, which we refer to as the licensed territory. We also granted Sarepta an option to expand the licensed territory to include specified countries in Central and South America. We have retained commercialization rights in the rest of the world including the United States.

If ezutromid receives marketing approval, we intend to commercialize it initially in the United States with our own focused, specialized sales force that we plan to establish. We believe that medical specialists treating DMD are sufficiently concentrated that we will be able to effectively promote ezutromid with a targeted sales team in the United States. We also believe that our relationships with patient advocacy groups will strengthen our ability to market ezutromid. Outside of the United States in the territories where we currently retain commercialization rights, we plan to evaluate the relative mertis of marketing ezutromid ourselves and utilizing collaboration, distribution and other marketing arrangements with third parties to commercialize ezutromid.

We plan to evaluate our options for maximizing the commercial opportunity for ridinilazole. We may determine to commercialize the product directly in the United States and Europe with our own specialized sales force or seek third-party collaborators for the commercialization of ridinilazole. We intend to evaluate the relative merits of retaining commercialization rights for ourselves or entering into collaboration arrangements with third parties depending on factors such as the anticipated development costs required to achieve marketing approval, the sales and marketing resources required in each territory in which we receive approval, the relative size of the market opportunity in such territory, the particular expertise of the third party and the proposed financial terms of the arrangement.

We are also in the process of building key capabilities, such as marketing, market access, sales management and medical affairs, to implement marketing and medical strategies for any products that we market through our own sales organization and to oversee and support our sales force. The responsibilities of the marketing organization would include developing educational initiatives with respect to approved products and expanding relationships with thought leaders in relevant fields of medicine.

Government Regulation

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, packaging, storage, recordkeeping, labeling, advertising,

promotion, distribution, marketing, post-approval monitoring and reporting, and import and export of pharmaceutical products. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

Review and Approval of Drugs in the United States

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and implementing regulations. The failure to comply with the FDCA and applicable U.S. requirements at any time during the product development process, approval process or after approval may subject an applicant or sponsor to a variety of administrative or judicial sanctions, including refusal by the FDA to approve pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters and other types of letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement of profits, or civil or criminal investigations and penalties brought by the FDA and the Department of Justice, or DOJ, or other federal and state governmental entities.

An applicant seeking approval to market and distribute a new drug product in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;
- submission to the FDA of an IND, which must take effect before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with current good clinical practices, or GCP, to establish the safety and efficacy of the proposed drug product for each indication;
- preparation and submission to the FDA of a new drug application, or NDA;
- review of the product candidate by an FDA advisory committee, where appropriate or if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with current Good Manufacturing Practices, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data;
- payment of user fees and securing FDA approval of the NDA; and
- compliance with any post-approval requirements, including Risk Evaluation and Mitigation Strategies, or REMS, where applicable, and any post-approval studies required by the FDA.

Preclinical Studies

Preclinical studies include laboratory evaluation of the purity and stability of the manufactured drug substance or API and the formulated drug or drug product, as well as *in vitro* and animal studies to assess the safety and activity of the drug for initial testing in humans and to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations. The results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, are submitted to the FDA as part of an IND. Some

long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted.

Companies usually must complete some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, and must also develop additional information about the chemistry and physical characteristics of the investigational product and finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the candidate product and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the candidate product does not undergo unacceptable deterioration over its shelf life.

The IND and IRB Processes

An IND is an exemption from the FDCA that allows an unapproved drug to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer an investigational drug to humans. Such authorization must be secured prior to interstate shipment and administration of any new drug that is not the subject of an approved NDA. In support of a request for an IND, applicants must submit a protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, are submitted to the FDA as part of an IND. The FDA requires a 30-day waiting period after the filing of each IND before clinical trials may begin. This waiting period is designed to allow the FDA to review the IND to determine whether human research subjects will be exposed to unreasonable health risks. At any time during this 30-day period, the FDA may raise concerns or questions about the conduct of the trials as outlined in the IND and impose a clinical hold. In this case, the IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can begin.

Following commencement of a clinical trial under an IND, the FDA may also place a clinical hold or partial clinical hold on that trial. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing investigation. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND. For example, a specific protocol or part of a protocol is not allowed to proceed, while other protocols may do so. No more than 30 days after imposition of a clinical hold or partial clinical hold, the FDA will provide the sponsor a written explanation of the basis for the hold. Following issuance of a clinical hold or partial clinical hold, an investigation may only resume after the FDA has notified the sponsor that the investigation may proceed. The FDA will base that determination on information provided by the sponsor correcting the deficiencies previously cited or otherwise satisfying the FDA that the investigation can proceed.

A sponsor may choose, but is not required, to conduct a foreign clinical study under an IND. When a foreign clinical study is conducted under an IND, all FDA IND requirements must be met unless waived. When the foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with FDA certain regulatory requirements in order to use the study as support for an IND or application for marketing approval. Specifically, on April 28, 2008, the FDA amended its regulations governing the acceptance of foreign clinical studies not conducted under an investigational new drug application as support for an IND or a new drug application. The final rule provides that such studies must be conducted in accordance with good clinical practice, or GCP, including review and approval by an independent ethics committee, or IEC, and informed consent from subjects. The GCP requirements in the final rule encompass both ethical and data integrity standards for clinical studies. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical studies, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign studies are conducted in a manner comparable to that required for IND studies.

In addition to the foregoing IND requirements, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB

must conduct continuing review and reapprove the study at least annually. The IRB must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects. An IRB must operate in compliance with FDA regulations. An IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the product candidate has been associated with unexpected serious harm to patients.

Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a trial may move forward at designated check points based on access that only the group maintains to available data from the study. Suspension or termination of development during any phase of clinical trials can occur if it is determined that the participants or patients are being exposed to an unacceptable health risk. Other reasons for suspension or termination may be made by us based on evolving business objectives and/or competitive climate.

Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health, or NIH, for public dissemination on its ClinicalTrials.gov website.

Human Clinical Trials in Support of an NDA

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Clinical trials are conducted under written study protocols detailing, among other things, the inclusion and exclusion criteria, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

Phase 1. The drug is initially introduced into healthy human subjects or, in certain indications such as cancer, patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness and to determine optimal dosage.

Phase 2. The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.

Phase 3. The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. In addition, IND safety reports must be submitted to the FDA for any of the following: serious and unexpected suspected adverse reactions; findings from other studies or animal or *in vitro* testing that suggest a significant risk in humans exposed to the drug; and any clinically important increase in the case of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has

been associated with unexpected serious harm to patients. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted.

Concurrent with clinical trials, companies often complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other things, must develop methods for testing the identity, strength, quality, purity, and potency of the final drug. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

Submission of an NDA to the FDA

Assuming successful completion of required clinical testing and other requirements, the results of the preclinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the drug product for one or more indications. Under federal law, the submission of most NDAs is subject to an application user fee, which for the federal fiscal year 2017 is $2,038,100. The sponsor of an approved NDA is also subject to annual product and establishment user fees, which for fiscal year 2017 are $97,750 per product and $512,200 per establishment. Certain exceptions and waivers are available for some of these fees, such as an exception from the application fee for drugs with orphan designation and a waiver for certain small businesses, an exception from the establishment fee when the establishment does not engage in manufacturing the drug during a particular fiscal year and an exception from the product fee for a drug that is the same as another drug approved under an abbreviated pathway.

The FDA conducts a preliminary review of an NDA within 60 calendar days of its receipt and strives to inform the sponsor by the 74th day after the FDA's receipt of the submission whether the application is sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review.

The FDA has agreed to certain performance goals in the review process of NDAs. Under that agreement, 90% of applications seeking approval of new molecular entities, or NMEs, are meant to be reviewed within ten months from the date on which FDA accepts the NDA for filing, and 90% of applications for NMEs that have been designated for "priority review" are meant to be reviewed within six months of the filing date. For applications seeking approval of drugs that are not NMEs, the ten-month and six-month review periods run from the date that FDA receives the application. The review process may be extended by the FDA for three additional months to consider new information or clarification provided by the applicant to address an outstanding deficiency identified by the FDA following the original submission.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is or will be manufactured. These pre-approval inspections cover all facilities associated with an NDA submission, including drug component manufacturing (such as active pharmaceutical ingredients), finished drug product manufacturing, and control testing laboratories. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP.

In addition, as a condition of approval, the FDA may require an applicant to develop a REMS. REMS use risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely

to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events, and whether the product is a new molecular entity. REMS can include medication guides, physician communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU may include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The FDA may require a REMS before approval or post-approval if it becomes aware of a serious risk associated with use of the product. The requirement for a REMS can materially affect the potential market and profitability of a product.

The FDA may refer an application for a novel drug to an advisory committee or explain why such referral was not made. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Fast Track, Breakthrough Therapy,Priority Review and Regenerative Advanced Therapy Designations

The FDA is authorized to designate certain products for expedited review if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition. These programs are referred to as fast track designation, breakthrough therapy designation, priority review designation and regenerative advanced therapy designation.

Specifically, the FDA may designate a product for fast track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For fast track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a fast track product's application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a fast track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. However, the FDA's time period goal for reviewing a fast track application does not begin until the last section of the application is submitted. In addition, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Second, a product may be designated as a breakthrough therapy if it is intended, either alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA may take certain actions with respect to breakthrough therapies, including holding meetings with the sponsor throughout the development process; providing timely advice to the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; and taking other steps to design the clinical trials in an efficient manner.

Third, the FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The FDA determines, on a case- by-case basis, whether the proposed product represents a significant improvement when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting product reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, and evidence of safety and effectiveness in a new subpopulation. A priority designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on a marketing application from ten months to six months.

Finally, with passage of the 21st Century Cures Act, or Cures Act, in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

Accelerated Approval Pathway

The FDA may grant accelerated approval to a drug for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a drug when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, or IMM, and that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Drugs granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. An intermediate clinical endpoint is a measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a drug, such as an effect on IMM. The FDA has limited experience with accelerated approvals based on intermediate clinical endpoints, but has indicated that such endpoints generally may support accelerated approval where the therapeutic effect measured by the endpoint is not itself a clinical benefit and basis for traditional approval, if there is a basis for concluding that the therapeutic effect is reasonably likely to predict the ultimate clinical benefit of a drug.

The accelerated approval pathway is most often used in settings in which the course of a disease is long and an extended period of time is required to measure the intended clinical benefit of a drug, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. For example, accelerated approval has been used extensively in the development and approval of drugs for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large clinical trials to demonstrate a clinical or survival benefit.

The accelerated approval pathway is usually contingent on a sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's clinical benefit. As a result, a drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

Limited Population Antibacterial Drug Pathway

With passage of the Cures Act, Congress authorized the FDA to approve an antibacterial or antifungal drug, alone or in combination with one or more other drugs, as a "limited population drug." To qualify for this approval pathway, the drug must be intended to treat a serious or life-threatening infection in a limited population of patients with unmet needs; the standards for approval of drugs and biologics under the FDCA and the Public

Health Service Act, or PHSA, must be satisfied; and the FDA must receive a written request from the sponsor to approve the drug as a limited population drug pursuant to this provision. The FDA's determination of safety and effectiveness for such a product must reflect the benefit-risk profile of such drug in the intended limited population, taking into account the severity, rarity, or prevalence of the infection the drug is intended to treat and the availability or lack of alternative treatment in such a limited population.

Any drug or biologic approved under this pathway must be labeled with the statement "Limited Population" in a prominent manner and adjacent to the proprietary name of the drug or biological product. The prescribing information must also state that the drug is indicated for use in a limited and specific population of patients and copies of all promotional materials relating to the drug must be submitted to the FDA at least 30 days prior to dissemination of the materials. If the FDA subsequently approves the drug for a broader indication, the agency may remove any post-marketing conditions, including requirements with respect to labeling and review of promotional materials applicable to the product. Nothing in this pathway to approval of a limited population drug prevents sponsors of such products from seeking designation or approval under other provisions of the FDCA, such as accelerated approval.

The FDA's Decision on an NDA

On the basis of the FDA's evaluation of the NDA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

If the FDA approves a product, it may limit the approved indications for use for the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess the drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, many types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being

implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, suspension of the approval, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, as well as the Drug Supply Chain Security Act, or DSCA, which regulate the distribution and tracing of prescription drugs and prescription drug samples at the federal level, and set minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution. The DSCA imposes requirements to ensure accountability in distribution and to identify and remove counterfeit and other illegitimate products from the market.

Abbreviated New Drug Applications for Generic Drugs

In 1984, with passage of the Hatch-Waxman Amendments to the FDCA, Congress authorized the FDA to approve generic drugs that are the same as drugs previously approved by the FDA under the NDA provisions of the statute. To obtain approval of a generic drug, an applicant must submit an abbreviated new drug application, or ANDA, to the agency. In support of such applications, a generic manufacturer may rely on the preclinical and clinical testing previously conducted for a drug product previously approved under an NDA, known as the reference listed drug, or RLD.

Specifically, in order for an ANDA to be approved, the FDA must find that the generic version is identical to the RLD with respect to the active ingredients, the route of administration, the dosage form, and the strength of the drug. At the same time, the FDA must also determine that the generic drug is "bioequivalent" to the innovator drug. Under the statute, a generic drug is bioequivalent to a RLD if "the rate and extent of absorption of the drug do not show a significant difference from the rate and extent of absorption of the listed drug."

Upon approval of an ANDA, the FDA indicates whether the generic product is "therapeutically equivalent" to the RLD in its publication "Approved Drug Products with Therapeutic Equivalence Evaluations," also referred to as the "Orange Book." Physicians and pharmacists consider a therapeutic equivalent generic drug to be fully substitutable for the RLD. In addition, by operation of certain state laws and numerous health insurance programs, the FDA's designation of therapeutic equivalence often results in substitution of the generic drug without the knowledge or consent of either the prescribing physician or patient.

Under the Hatch-Waxman Amendments, the FDA may not approve an ANDA until any applicable period of non-patent exclusivity for the RLD has expired. The FDCA provides a period of five years of non-patent data exclusivity for a new drug containing a new chemical entity. A new chemical entity is a drug that contains no active moiety that has previously been approved by the FDA in any other NDA. An active moiety is the molecule or ion responsible for the physiological or pharmacological action of the drug substance. In cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification, in which case the applicant may submit its application four years following the original product approval. The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication.

Hatch-Waxman Patent Certification and the 30-Month Stay

Upon approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or an approved method of using the product. Each of the patents listed by the NDA sponsor is published in the Orange Book. When an ANDA applicant files its application with the FDA, the applicant is required to certify to the FDA concerning any patents listed for the reference product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval. To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would.

Specifically, the applicant must certify with respect to each patent that:

- the required patent information has not been filed;
- the listed patent has expired;
- the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or
- the listed patent is invalid, unenforceable or will not be infringed by the new product.

A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a "Paragraph IV certification." If the applicant does not challenge the listed patents or indicates that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired (other than method of use patents involving indications for which the ANDA applicant is not seeking approval).

If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months after the receipt of the Paragraph IV notice, expiration of the patent or a decision in the infringement case that is favorable to the ANDA applicant.

Pediatric Studies and Exclusivity

Under the Pediatric Research Equity Act of 2003, an NDA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the drug product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. With enactment of the Food and Drug Administration Safety and Innovation Act, or FDASIA, in 2012, sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in FDASIA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if an NDA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application.

Orphan Drug Designation and Exclusivity

The FDA has granted orphan drug designation to ezutromid for the treatment of DMD. Under the Orphan Drug Act, the FDA may designate a drug product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a drug product available in the United States for treatment of the disease or condition will be recovered from sales of the product). A company must request orphan product designation before submitting an NDA. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will be receiving orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications for the same product for the same indication for seven years, except in certain limited circumstances. Competitors may receive approval of different products for the indication for which the orphan product has exclusivity and may obtain approval for the same product but for a different indication. If a drug or drug product designated as an orphan product ultimately receives marketing approval for an indication broader than what was designated in its orphan product application, it may not be entitled to exclusivity.

Rare Pediatric Disease Priority Review Voucher Program

With enactment of the FDASIA in 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications that meet the criteria specified in the law. This provision is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application.

For the purposes of this program, a "rare pediatric disease" is a (a) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (b) rare disease or conditions within the meaning of the Orphan Drug Act. A sponsor may choose to request rare pediatric disease designation, but the designation process is entirely voluntary; requesting designation is not a prerequisite to requesting or receiving a priority review voucher. In addition, sponsors who choose not to submit a rare pediatric disease designation request may nonetheless receive a priority review voucher if they request such a voucher in their original marketing application and meet all of the eligibility criteria. Under the Cures Act, the Rare Pediatric Disease Priority Review Voucher program was reauthorized until 2020. However, if a drug is designated before October 1, 2020, it is eligible to receive a voucher if approved before October 2022.

GAIN Exclusivity for Antibiotics

The FDA has designated ridinilazole as a qualified infectious disease product, or QIDP, under the Generating Antibiotic Incentives Now Act, or GAIN Act. Congress passed this legislation to encourage the development of antibacterial and antifungal drug products that treat pathogens that cause serious and life-threatening infections. To that end, the GAIN Act grants an additional five years of exclusivity upon the approval of an NDA for a drug product designated by the FDA as a QIDP. Thus, for a QIDP, the periods of five-year new chemical entity exclusivity, three-year new clinical investigation exclusivity and seven-year orphan drug exclusivity, would become ten years, eight years and 12 years, respectively.

A QIDP is defined in the GAIN Act to mean "an antibacterial or antifungal drug for human use intended to treat serious or life-threatening infections, including those caused by—(1) an antibacterial or antifungal resistant pathogen, including novel or emerging infectious pathogens;" or (2) certain "qualifying pathogens." A "qualifying pathogen" is a pathogen that has the potential to pose a serious threat to public health (such as resistant gram positive pathogens, multi-drug resistant gram negative bacteria, multi-drug resistant tuberculosis and *Clostridium difficile*) and that is included in a list established and maintained by the FDA. A drug sponsor may request the FDA to designate its product as a QIDP any time before the submission of an NDA. The FDA must make a QIDP determination within 60 days of the designation request. A product designated as a QIDP will be granted priority review by FDA and can qualify for "fast track" status.

The additional five years of exclusivity under the GAIN Act for drug products designated by the FDA as QIDPs applies only to a drug that is first approved on or after July 9, 2012. Additionally, the five year exclusivity extension does not apply to: a supplement to an application under FDCA Section 505(b) for any QIDP for which an extension is in effect or has expired; a subsequent application filed with respect to a product approved by the FDA for a change that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength; or a product that does not meet the definition of a QIDP under Section 505(g) based upon its approved uses.

Patent Term Restoration and Extension

The term of a U.S. patent that covers a drug, biological product or medical device approved pursuant to a PMA may also be eligible for patent term extension when FDA approval is granted, provided that certain statutory and regulatory requirements are met. The length of the patent term extension is related to the length of time the drug is under regulatory review while the patent is in force. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration date set for the patent. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be granted an extension and only those claims reading on the approved drug may be extended. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug, provided that statutory and regulatory requirements are met. The USPTO reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

The 21st Century Cures Act

On December 13, 2016, President Obama signed the 21st Century Cures Act, or Cures Act, into law. The Cures Act is designed to modernize and personalize healthcare, spur innovation and research, and streamline the discovery and development of new therapies through increased federal funding of particular programs. It authorizes increased funding for the FDA to spend on innovation projects. The new law also amends the Public Health Service Act to reauthorize and expand funding for the National Institutes of Health. The Act establishes the NIH Innovation Fund to pay for the cost of development and implementation of a strategic plan, early stage investigators and research. It also charges NIH with leading and coordinating expanded pediatric research. Further, the Cures Act directs the Centers for Disease Control and Prevention to expand surveillance of neurological diseases.

With amendments to the FDCA and PHSA, Title III of the Cures Act seeks to accelerate the discovery, development, and delivery of new medicines and medical technologies. To that end, and among other provisions, the Cures Act reauthorizes the existing priority review voucher program for certain drugs intended to treat rare pediatric diseases until 2020; creates a new priority review voucher program for drug applications determined to be material national security threat medical countermeasure applications; revises the FDCA to streamline review of combination product applications; requires FDA to evaluate the potential use of "real world evidence" to help support approval of new indications for approved drugs; provides a new "limited population" approval pathway for antibiotic and antifungal drugs intended to treat serious or life-threatening infections; and authorizes FDA to designate a drug as a "regenerative advanced therapy," thereby making it eligible for certain expedited review and approval designations.

Regulation Outside the United States

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of drug products. Whether or not it obtains FDA approval for a product, the company would need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. The approval process ultimately varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Regulation and Marketing Authorization in the European Union

The process governing approval of medicinal products in the European Union follows essentially the same lines as in the United States and, likewise, generally involves satisfactorily completing each of the following:

- preclinical laboratory tests, animal studies and formulation studies all performed in accordance with the applicable E.U. Good Laboratory Practice regulations;
- submission to the relevant national authorities of a clinical trial application, or CTA, which must be approved before human clinical trials may begin;
- performance of adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication;
- submission to the relevant competent authorities of a marketing authorization application, or MAA, which includes the data supporting safety and efficacy as well as detailed information on the manufacture and composition of the product in clinical development and proposed labelling;
- satisfactory completion of an inspection by the relevant national authorities of the manufacturing facility or facilities, including those of third parties, at which the product is produced to assess compliance with strictly enforced current cGMP;
- potential audits of the non-clinical and clinical trial sites that generated the data in support of the MAA; and
- review and approval by the relevant competent authority of the MAA before any commercial marketing, sale or shipment of the product.

Preclinical Studies

Preclinical tests include laboratory evaluations of product chemistry, formulation and stability, as well as studies to evaluate toxicity in animal studies, in order to assess the potential safety and efficacy of the product. The conduct of the preclinical tests and formulation of the compounds for testing must comply with the relevant E.U. regulations and requirements. The results of the preclinical tests, together with relevant manufacturing information and analytical data, are submitted as part of the CTA.

Clinical Trial Approval

Requirements for the conduct of clinical trials in the European Union including Good Clinical Practice, or GCP, are implemented in the Clinical Trials Directive 2001/20/EC and the GCP Directive 2005/28/EC. Pursuant to Directive 2001/20/EC and Directive 2005/28/EC, as amended, a system for the approval of clinical trials in the European Union has been implemented through national legislation of the member states. Under this system, approval must be obtained from the competent national authority of an E.U. member state in which a study is planned to be conducted, or in multiple member states if the clinical trial is to be conducted in a number of member states. To this end, a CTA is submitted, which must be supported by an investigational medicinal product dossier, or IMPD, and further supporting information prescribed by Directive 2001/20/EC and Directive 2005/28/EC and other applicable guidance documents. Furthermore, a clinical trial may only be started after a competent ethics committee has issued a favorable opinion on the clinical trial application in that country.

In April 2014, the E.U. legislature passed the new Clinical Trials Regulation, (EU) No 536/2014 (Clinical Trials Regulation), which will replace the current Clinical Trials Directive 2001/20/EC. To ensure that the rules for clinical trials are identical throughout the European Union, the new E.U. clinical trials legislation was passed as a regulation that is directly applicable in all E.U. member states. All clinical trials performed in the European Union are required to be conducted in accordance with the Clinical Trials Directive 2001/20/EC until the new Clinical Trials Regulation becomes applicable. According to the current plans of EMA, the new Clinical Trials Regulation will become applicable in October 2018. The Clinical Trials Directive 2001/20/EC will however still

apply three years from the entry into application of the Clinical Trials Regulation to (i) clinical trials applications submitted before the entry into application and (ii) clinical trials applications submitted within one year after the entry into application if the sponsor opts for old system.

The new Clinical Trials Regulation aims to simplify and streamline the approval of clinical trials in the European Union. The main characteristics of the regulation include:

- A streamlined application procedure via a single entry point, the E.U. portal.
- A single set of documents to be prepared and submitted for the application as well as simplified reporting procedures that will spare sponsors from submitting broadly identical information separately to various bodies and different member states.
- A harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts. Part I is assessed jointly by all member states concerned. Part II is assessed separately by each member state concerned.
- Strictly defined deadlines for the assessment of clinical trial application.
- The involvement of the ethics committees in the assessment procedure in accordance with the national law of the member state concerned but within the overall timelines defined by the Clinical Trials Regulation.

Marketing Authorization

Authorization to market a product in the member states of the European Union proceeds under one of four procedures: a centralized authorization procedure, a mutual recognition procedure, a decentralized procedure or a national procedure.

Centralized Authorization Procedure

The centralized procedure enables applicants to obtain a marketing authorization that is valid in all E.U. member states based on a single application. Certain medicinal products, including products developed by means of biotechnological processes, must undergo the centralized authorization procedure for marketing authorization, which, if granted by the European Commission, is automatically valid in all 28 E.U. member states. The EMA and the European Commission administer this centralized authorization procedure pursuant to Regulation (EC) No 726/2004.

Pursuant to Regulation (EC) No 726/2004, this procedure is mandatory for:

- medicinal products developed by means of one of the following biotechnological processes:
  - recombinant DNA technology;
  - controlled expression of genes coding for biologically active proteins in prokaryotes and eukaryotes including transformed mammalian cells; and
  - hybridoma and monoclonal antibody methods;
- advanced therapy medicinal products as defined in Article 2 of Regulation (EC) No. 1394/2007 on advanced therapy medicinal products;
- medicinal products for human use containing a new active substance that, on the date of effectiveness of this regulation, was not authorized in the European Union, and for which the therapeutic indication is the treatment of any of the following diseases:
  - acquired immune deficiency syndrome;
  - cancer;

- neurodegenerative disorder;
- diabetes;
- auto-immune diseases and other immune dysfunctions; and
- viral diseases; and

- medicinal products that are designated as orphan medicinal products pursuant to Regulation (EC) No 141/2000.

The centralized authorization procedure is optional for other medicinal products if they contain a new active substance or if the applicant shows that the medicinal product concerned constitutes a significant therapeutic, scientific or technical innovation or that the granting of authorization is in the interest of patients in the European Union.

Administrative Procedure

Under the centralized authorization procedure, the EMA's Committee for Human Medicinal Products, or CHMP, serves as the scientific committee that renders opinions about the safety, efficacy and quality of medicinal products for human use on behalf of the EMA. The CHMP is composed of experts nominated by each member state's national authority for medicinal products, with expert appointed to act as Rapporteur for the coordination of the evaluation with the possible assistance of a further member of the Committee acting as a Co-Rapporteur. After approval, the Rapporteur(s) continue to monitor the product throughout its life cycle. The CHMP has 210 days to adopt an opinion as to whether a marketing authorization should be granted. The process usually takes longer in case additional information is requested, which triggers clock-stops in the procedural timelines. The process is complex and involves extensive consultation with the regulatory authorities of member states and a number of experts. When an application is submitted for a marketing authorization in respect of a drug that is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation, the applicant may pursuant to Article 14(9) Regulation (EC) No 726/2004 request an accelerated assessment procedure. If the CHMP accepts such request, the time-limit of 210 days will be reduced to 150 days but it is possible that the CHMP can revert to the standard time-limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment. Once the procedure is completed, a European Public Assessment Report, or EPAR, is produced. If the opinion is negative, information is given as to the grounds on which this conclusion was reached. After the adoption of the CHMP opinion, a decision on the MAA must be adopted by the European Commission, after consulting the E.U. member states, which in total can take more than 60 days.

Conditional Approval

In specific circumstances, E.U. legislation (Article 14(7) Regulation (EC) No 726/2004 and Regulation (EC) No 507/2006 on Conditional Marketing Authorisations for Medicinal Products for Human Use) enables applicants to obtain a conditional marketing authorization prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional approvals may be granted for product candidates (including medicines designated as orphan medicinal products) if (1) the risk-benefit balance of the product candidate is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) the product fulfills unmet medical needs and (4) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization.

Marketing Authorization under Exceptional Circumstances

Under Article 14(8) Regulation (EC) No 726/2004, products for which the applicant can demonstrate that comprehensive data (in line with the requirements in Annex I of Directive 2001/83/EC, as amended) cannot be provided (due to specific reasons foreseen in the legislation) might be eligible for marketing authorization under exceptional circumstances. This type of authorization is reviewed annually to reassess the risk-benefit balance. The fulfillment of any specific procedures/obligations imposed as part of the marketing authorization under exceptional circumstances is aimed at the provision of information on the safe and effective use of the product and will normally not lead to the completion of a full dossier/approval.

Market Authorizations Granted by Authorities of E.U. Member States

In general, if the centralized procedure is not followed, there are three alternative procedures as prescribed in Directive 2001/83/EC:

- The decentralized procedure allows applicants to file identical applications to several E.U. member states and receive simultaneous national approvals based on the recognition by E.U. member states of an assessment by a reference member state.
- The national procedure is only available for products intended to be authorized in a single E.U. member state.
- A mutual recognition procedure similar to the decentralized procedure is available when a marketing authorization has already been obtained in at least one E.U. member state.

A marketing authorization may be granted only to an applicant established in the European Union.

Pediatric Studies

Prior to obtaining a marketing authorization in the European Union, applicants have to demonstrate compliance with all measures included in an EMA-approved Paediatric Investigation Plan, or PIP, covering all subsets of the pediatric population, unless the EMA has granted a product-specific waiver, a class waiver, or a deferral for one or more of the measures included in the PIP. The respective requirements for all marketing authorization procedures are set forth in Regulation (EC) No 1901/2006, which is referred to as the Pediatric Regulation. This requirement also applies when a company wants to add a new indication, pharmaceutical form or route of administration for a medicine that is already authorized. The Pediatric Committee of the EMA, or PDCO, may grant deferrals for some medicines, allowing a company to delay development of the medicine in children until there is enough information to demonstrate its effectiveness and safety in adults. The PDCO may also grant waivers when development of a medicine in children is not needed or is not appropriate, such as for diseases that only affect the elderly population.

Before a marketing authorization application can be filed, or an existing marketing authorization can be amended, the EMA determines that companies actually comply with the agreed studies and measures listed in each relevant PIP.

Periods of Authorization and Renewals

A marketing authorization is valid for five years in principle and the marketing authorization may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA or by the competent authority of the authorizing member state. To this end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing authorization was granted, at least six months before the marketing authorization ceases to be valid. Once renewed, the marketing authorization is valid for an unlimited period, unless the European Commission or the competent authority decides, on justified grounds relating to

pharmacovigilance, to proceed with one additional five-year renewal. Any authorization which is not followed by the actual placing of the drug on the E.U. market (in case of centralized procedure) or on the market of the authorizing member state within three years after authorization ceases to be valid (the so-called sunset clause).

Orphan Drug Designation and Exclusivity

The European Commission, following an evaluation by the EMA's Committee for Orphan Medicinal Products, has designated ezutromid as an orphan medicinal product (EU orphan designation number: EU/3/08/591). Pursuant to Regulation (EC) No 141/2000 and Regulation (EC) No. 847/2000, the European Commission can grant such orphan medicinal product designation to products for which the sponsor can establish that it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 people in the European Union, or a life threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that sales of the drug in the European Union would generate a sufficient return to justify the necessary investment. In addition, the sponsor must establish that there is no other satisfactory method approved in the European Union of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients.

Orphan drug designation is not a marketing authorization. It is a designation that provides a number of benefits, including fee reductions, regulatory assistance, and the possibility to apply for a centralized E.U. marketing authorization, as well as ten years of market exclusivity following a marketing authorization. During this market exclusivity period, neither the EMA, the European Commission nor the member states can accept an application or grant a marketing authorization for a "similar medicinal product." A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as those contained in an authorized orphan medicinal product and that is intended for the same therapeutic indication. The market exclusivity period for the authorized therapeutic indication may be reduced to six years if, at the end of the fifth year, it is established that the orphan designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. In addition, a competing similar medicinal product may in limited circumstances be authorized prior to the expiration of the market exclusivity period, including if it is shown to be safer, more effective or otherwise clinically superior to the already approved orphan drug. Furthermore, a product can lose orphan designation, and the related benefits, prior to us obtaining a marketing authorization if it is demonstrated that the orphan designation criteria are no longer met.

Regulatory Data Protection

E.U. legislation also provides for a system of regulatory data and market exclusivity. According to Article 14(11) of Regulation (EC) No 726/2004, as amended, and Article 10(1) of Directive 2001/83/EC, as amended, upon receiving marketing authorization, new chemical entities approved on the basis of complete independent data package benefit from eight years of data exclusivity and an additional two years of market exclusivity. Data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic (abbreviated) application. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator's data may be referenced, but no generic medicinal product can be marketed until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten years, the marketing authorization holder, or MAH, obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity and the innovator is able to gain the period of data exclusivity, another company nevertheless could also market another version of the drug if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical test, preclinical tests and clinical trials. However, products designated as orphan medicinal products enjoy, upon receiving marketing authorization, a period of ten years of orphan market exclusivity—see also *Orphan Drug Designation and Exclusivity*. Depending upon the timing and duration of the E.U. marketing

authorization process, products may be eligible for up to five years' supplementary protection certificates, or SPCs, pursuant to Regulation (EC) No 469/2009. Such SPCs extend the rights under the basic patent for the drug.

Regulatory Requirements After a Marketing Authorization has been Obtained

If we obtain authorization for a medicinal product in the European Union, we will be required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products:

Pharmacovigilance and other requirements

We will, for example, have to comply with the E.U.'s stringent pharmacovigilance or safety reporting rules, pursuant to which post-authorization studies and additional monitoring obligations can be imposed. Other requirements relate, for example, to the manufacturing of products and APIs in accordance with good manufacturing practice standards. E.U. regulators may conduct inspections to verify our compliance with applicable requirements, and we will have to continue to expend time, money and effort to remain compliant. Non-compliance with E.U. requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties in the European Union. Similarly, failure to comply with the E.U.'s requirements regarding the protection of individual personal data can also lead to significant penalties and sanctions. Individual E.U. member states may also impose various sanctions and penalties in case we do not comply with locally applicable requirements.

Manufacturing

The manufacturing of authorized drugs, for which a separate manufacturer's license is mandatory, must be conducted in strict compliance with the EMA's Good Manufacturing Practices, or GMP, requirements and comparable requirements of other regulatory bodies in the European Union, which mandate the methods, facilities and controls used in manufacturing, processing and packing of drugs to assure their safety and identity. The EMA enforces its current GMP requirements through mandatory registration of facilities and inspections of those facilities. The EMA may have a coordinating role for these inspections while the responsibility for carrying them out rests with the member states competent authority under whose responsibility the manufacturer falls. Failure to comply with these requirements could interrupt supply and result in delays, unanticipated costs and lost revenues, and could subject the applicant to potential legal or regulatory action, including but not limited to warning letters, suspension of manufacturing, seizure of product, injunctive action or possible civil and criminal penalties.

Marketing and Promotion

The marketing and promotion of authorized drugs, including industry-sponsored continuing medical education and advertising directed toward the prescribers of drugs and/or the general public, are strictly regulated in the European Union under Directive 2001/83/EC. The applicable regulations aim to ensure that information provided by holders of marketing authorizations regarding their products is truthful, balanced and accurately reflects the safety and efficacy claims authorized by the EMA or by the competent authority of the authorizing member state. Failure to comply with these requirements can result in adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties.

Patent Term Extension

In order to compensate the patentee for delays in obtaining a marketing authorization for a patented product, a supplementary certificate, or SPC, may be granted extending the exclusivity period for that specific product by up to five years. Applications for SPCs must be made to the relevant patent office in each E.U. member state and the granted certificates are valid only in the member state of grant. An application has to be made by the patent

owner within six months of the first marketing authorization being granted in the European Union (assuming the patent in question has not expired, lapsed or been revoked) or within six months of the grant of the patent (if the marketing authorization is granted first). In the context of SPCs, the term "product" means the active ingredient or combination of active ingredients for a medicinal product and the term "patent" means a patent protecting such a product or a new manufacturing process or application for it. The duration of an SPC is calculated as the difference between the patent's filing date and the date of the first marketing authorization, minus five years, subject to a maximum term of five years.

A six month pediatric extension of an SPC may be obtained where the patentee has carried out an agreed pediatric investigation plan, the authorized product information includes information on the results of the studies and the product is authorized in all member states of the European Union.

Possible Change of Regulatory Framework in United Kingdom as a result of the Brexit

On June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union (commonly referred to as "Brexit"). The withdrawal of the U.K. from the European Union will take effect either on the effective date of the withdrawal agreement or, in the absence of agreement, two years after the United Kingdom provides a notice of withdrawal pursuant to Article 50 of the E.U. Treaty, unless the European Council, in agreement with the United Kingdom, unanimously decides to extend this period. On March 29, 2017, the U.K. Prime Minister formally delivered the notice of withdrawal. Since the regulatory framework for pharmaceutical products in the U.K. covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products, is derived from European Union directives and regulations, the Brexit could materially impact the future regulatory regime which applies to our products candidates in the U.K., in particular the approval of any of our product candidates in the U.K. We do not know to what extent Brexit, or any resulting changes, would affect our ability to conduct clinical trials or obtain marketing approval in the U.K. for our product candidates, and each could materially impact our ability to conduct clinical trials or obtain marketing approval in the U.K. on a timely basis, or at all.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Sales of products will depend, in part, on the extent to which the costs of the products will be covered by third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the approved products for a particular indication. Additionally, the containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results.

In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable regulatory approvals. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Third-party reimbursement may not be sufficient to maintain price levels high enough to realize an appropriate return on investment in product development.

In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that drug products may be marketed only after a reimbursement price has been agreed. Some

countries may require the completion of additional studies that compare the cost-effectiveness of our drug candidate to currently available therapies (so called health technology assessment, or HTA) in order to obtain reimbursement or pricing approval. For example, the European Union provides options for its member states to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. E.U. member states may approve a specific price for a drug product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the drug product on the market. Other member states allow companies to fix their own prices for drug products, but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. The downward pressure on health care costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various E.U. member states, and parallel distribution (arbitrage between low-priced and high-priced member states), can further reduce prices. Any country that has price controls or reimbursement limitations for drug products may not allow favorable reimbursement and pricing arrangements.

Healthcare Law and Regulation

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of drug products that are granted marketing approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse and other healthcare laws and regulations. Such restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal False Claims Act imposes civil penalties, and provides for civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, including the final omnibus rule published in January 2013, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal Physician Payments Sunshine Act will require certain manufacturers of drugs, devices, biologics and medical supplies to report to the Centers for Medicare & Medicaid Services, or CMS, within the Department of Health and Human Services information related to payments and other transfers of value to physicians and teaching hospitals and physician ownership and investment interests held by physicians and their immediate family members; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Healthcare Reform

A primary trend in the United States healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of pharmaceutical and biopharmaceutical products, limiting coverage and reimbursement for drugs and other medical products, government control and other changes to the healthcare system in the United States.

In March 2010, the U.S. Congress enacted the Patient Protection and Affordable Care Act, or the ACA, which, among other things, includes changes to the coverage and payment for products under government health care programs. Among the provisions of the ACA of importance to potential drug candidates are:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, although this fee would not apply to sales of certain products approved exclusively for orphan indications;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- expanded manufacturers' rebate liability under the Medicaid Drug Rebate Program by increasing the minimum rebate for both branded and generic drugs and revising the definition of "average manufacturer price," or AMP, for calculating and reporting Medicaid drug rebates on outpatient prescription drug prices and extending rebate liability to prescriptions for individuals enrolled in Medicare Advantage plans;
- addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expanded the types of entities eligible for the 340B drug discount program;
- established the Medicare Part D coverage gap discount program by requiring manufacturers to provide a 50% point-of-sale-discount off the negotiated price of applicable brand drugs to eligible beneficiaries during their coverage gap period as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- the Independent Payment Advisory Board, or IPAB, which has authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs. However, the IPAB implementation has been not been clearly defined. The ACA provided that under certain circumstances, IPAB recommendations will become law unless Congress enacts legislation that will achieve the same or greater Medicare cost savings; and

- established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending. Funding has been allocated to support the mission of the Center for Medicare and Medicaid Innovation from 2011 to 2019.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

With the new administration and Congress, there will likely be additional legislative changes, including repeal and replacement of certain provisions of the ACA. In January 2017, Congress voted to adopt a budget resolution for fiscal year 2017, or the Budget Resolution, that authorizes the implementation of legislation that would repeal portions of the ACA. The Budget Resolution is not a law, however, it is widely viewed as the first step toward the passage of legislation that would repeal certain aspects of the ACA. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices.

The President and congressional leaders have expressed interest in repealing certain ACA provisions and replacing them with alternatives that may be less costly and provide state Medicaid programs and private health plans more flexibility. It is possible that these repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. The scope of potential future legislation to repeal and replace ACA provisions is highly uncertain in many respects, and it is possible that some of the ACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with ACA coverage expansion provisions. However, at this time the coverage expansion provisions of the ACA appear most likely to be repealed and replaced.

C. Organizational Structure

The following is a list of our subsidiaries:

Name of subsidiary	Country of registration	Activity	% holding
Summit Therapeutics Inc.	USA	Research and Development Services	100%
Summit Corporation Limited	England and Wales	Dormant	100%
Summit (Oxford) Limited	England and Wales	Research and Development	100%
Summit (Wales) Limited	England and Wales	Research and Development	100%
Summit (Cambridge) Limited	England and Wales	Dormant	100%
Summit Discovery 1 Limited	England and Wales	Dormant	100%
Summit Corporation Employee Benefit Trust Company Limited	England and Wales	Dormant	100%
MuOx Limited	England and Wales	Dormant	100%

D. Property, Plants and Equipment

Type/Uses	Location	Size	Lease Expiry
Executive office	Oxfordshire, United Kingdom	4,373 square feet	August 2017
Executive office	Oxfordshire, United Kingdom	6,781 square feet	February 2027
Executive office	Cambridge, Massachusetts	1,777 square feet	Rolling

Item 4A: Unresolved Staff Comments

Not applicable.

Item 5: Operating and Financial Review and Prospects

You should read the following discussion and analysis of our financial condition and results of operations together with "Selected Consolidated Financial Data" and our consolidated financial statements and the related notes thereto appearing at the end of this Annual Report. We present our consolidated financial statements in pounds sterling and in accordance with International Financial Reporting Standards, or IFRS, as issued by the International Accounting Standards Board, or IASB.

Some information included in this discussion and analysis, including statements regarding industry outlook, our expectations regarding our future performance, liquidity and capital resources and other statements regarding our plans and strategy for our business and related financing, are forward-looking statements. These forward-looking statements are subject to numerous risks and uncertainties. You should read the "Risk Factors" section of this Annual Report for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Solely for the convenience of the reader, unless otherwise indicated, all pound sterling amounts as of and for the year ended January 31, 2017 have been translated into U.S. dollars at the noon buying rate of the Federal Reserve Bank of New York on January 31, 2017, of £1.00 to $1.2585. These translations should not be considered representations that any such amounts have been, could have been or could be converted into U.S. dollars at that or any other exchange rate as of that or any other date.

Overview

We were founded in 2003 and are incorporated under the laws of England and Wales with the Registrar of Companies of England and Wales, United Kingdom. Our principal offices are located in the United Kingdom. Our ordinary shares have traded on AIM, which is a sub-market of the London Stock Exchange, since October 2004 and our American Depositary Shares, or ADSs, have traded on the NASDAQ Global Market since March 2015. Our historic business activities have included the research and development of drug candidates across a number of disease areas. We have also in the past provided drug discovery services to other pharmaceutical and biotechnology companies. However, we sold these drug discovery services businesses in 2009 as part of a broader restructuring initiative to focus on identifying and developing medicines in a range of major therapy areas. In 2012, we made a strategic decision to further refine our business focus and concentrate on the development of our clinical stage programs for Duchenne muscular dystrophy, or DMD, and *Clostridium difficile* infection, or CDI, in order to more efficiently capitalize on the scientific and commercial potential of these programs. Accordingly, we discontinued our in-house discovery efforts relating to the development of an iminosugar technology platform. We expanded our future generation utrophin modulator pipeline effort in November 2013 through the formation of a strategic alliance with the University of Oxford. As part of this transaction, we acquired an exclusive option to license intellectual property that is generated as part of our research with the University of Oxford in utrophin modulation. In 2014, we opened an office in Cambridge, Massachusetts, in order to strengthen our presence in the United States. We expect to undertake a significant proportion of our future development efforts for our clinical programs in the United States.

To date, we have financed our operations primarily through issuances of our ordinary shares and American Depositary Shares, or ADSs, payments to us under our license and collaboration agreement with Sarepta Therapeutics, Inc., or Sarepta, and development funding and other assistance from government entities, philanthropic, non-government and not for profit organizations and patient advocacy groups for our product candidates. In particular, we have received funding from the Wellcome Trust, Innovate UK, Joining Jack, the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, Charley's Fund, Cure Duchenne, Foundation to Eradicate Duchenne and the Nash Avery Foundation.

We have generated losses since inception. Our net loss was approximately £21.4 million for the year ended January 31, 2017, £20.1 million for the year ended January 31, 2016 (as adjusted) and £11.4 million for the year ended January 31, 2015 (as adjusted). As of January 31, 2017, we had an accumulated deficit of £73.8 million. We expect to incur significant expenses and increasing operating losses for at least the next several years in connection with conducting clinical trials for our lead product candidates, ezutromid (formerly SMT C1100) for the treatment of DMD and ridinilazole (formerly SMT19969) for the treatment of CDI, and seeking marketing approval for ezutromid in the United States and other territories where we retain commercialization rights and ridinilazole in the United States and the European Union as well as other geographies. In addition, subject to obtaining regulatory approval for ezutromid, ridinilazole or any of our future product candidates, we expect to incur significant commercialization expenses for product sales, marketing, distribution and outsourced manufacturing. We also incur additional costs associated with operating as a public company in the United States in addition to in the United Kingdom. Accordingly, we will need additional financing to support our continuing operations. Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

Operating Results

Important Financial and Operating Terms and Concepts

Revenue

Revenue currently consists of amounts received following the signing of an exclusive license and collaboration agreement with Sarepta Therapeutics, Inc., or Sarepta, in October 2016. Under the terms of the license and collaboration agreement, we received an upfront payment of $40.0 million (£32.8 million) from Sarepta. The terms of the license and collaboration agreement have been assessed, and we believe the development services to be indistinguishable and as a result the upfront payment has been initially reported as deferred income on the Consolidated Statement of Financial Position and is being recognized as revenue over the development period. We also will be eligible for future ezutromid-related development, regulatory and sales milestone payments totalling up to $522.0 million. This includes $42.0 million in respect of specified development milestones (including a $22.0 million milestone payment upon the first dosing of the last patient in our PhaseOut DMD trial, payable on or after April 1, 2017), $150.0 million in respect of specified regulatory milestones and $330.0 million from specified sales milestones. We are also eligible for escalating royalties ranging from a low to high teens percentage of net sales in the territories where we have granted Sarepta commercialization rights.

Other Operating Income

Other operating income includes income received and recognized from philanthropic, non-government and not for profit organizations and patient advocacy groups which are accounted for in accordance with IAS 20, "*Accounting for Government Grants and Disclosure of Government Assistance*." Amounts received through these sources are held as deferred income in the Consolidated Statement of Financial Position and are released to the Consolidated Statement of Comprehensive Income as the underlying expenditure is incurred and to the extent the conditions of the grant are met.

Operating Expenses

The majority of our operating expenses since inception have consisted of research and development activities and general and administrative costs.

Research and Development Expenses

Research and development expenses consist of all costs associated with our research and development activities.

These include:

- costs incurred in conducting our preclinical studies and clinical trials through contract research organizations, including preclinical toxicology, pharmacology, formulation and manufacturing work;
- employee related expenses, which include salary and benefits, for our research and development staff;
- costs associated with our strategic alliance with the University of Oxford; and
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies.

We utilize our employee and infrastructure resources across multiple research projects. We track expenses related to our clinical programs and certain preclinical programs on a per project basis. We expect our research and development expenses to continue to increase as compared to prior periods as we initiate and continue clinical trials of ezutromid for the treatment of DMD and ridinilazole for the treatment of CDI and continue our research activities and initiate preclinical programs for future product candidates. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs and product candidate manufacturing costs.

The table below summarizes our research and development expenses by category. Our DMD and CDI program expenses include costs paid to contract research organizations, manufacturing costs for our clinical trials, laboratory testing costs and research related expenses incurred in connection with our strategic alliance with the University of Oxford. Other research and development costs include staff and travel costs (including those of our internal DMD and CDI teams), research and development related legal costs, ongoing patent maintenance fees, an allocation of facility-related costs and historically non-core program related expenses.

	Year ended January 31,			
	2017	2017	2016	2015
		(in thousands)		
DMD program	$11,930	£ 9,480	£ 7,526	£ 4,719
CDI program	5,145	4,088	5,567	3,211
Other research and development costs	6,774	5,384	3,763	2,487
Total	$23,849	£18,952	£16,856	£10,417

From inception to January 31, 2017, our total DMD program expenses were £27.8 million and our total CDI program expenses were £17.8 million.

The successful development and commercialization of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the remainder of the development of ezutromid, ridinilazole or any of our future product candidates.

This is due to the numerous risks and uncertainties associated with product development and commercialization, including the uncertainty of:

- the progress, costs and results of clinical trials of ezutromid for DMD and ridinilazole for CDI;
- the scope, rate of progress, costs and results of preclinical development, laboratory testing and clinical trials for our future generation utrophin modulators;
- the costs, timing and outcome of regulatory review of our product candidates;
- the efficacy and potential advantages of our product candidates compared to alternative treatments, including any standard of care, and our ability to achieve market acceptance for any of our product candidates that receive marketing approval;
- the costs and timing of commercialization activities, including product sales, marketing, distribution and manufacturing, for any of our product candidates that receive marketing approval and the rate we expand our physical presence; and
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining, enforcing and protecting our intellectual property rights and defending against any intellectual property-related claims.

A change in the outcome of any of these variables with respect to the development of ezutromid or ridinilazole or any other future product candidate that we may develop could result in a significant change in the costs and timing associated with the development of that product candidate. For example, if the European Medicines Agency, or EMA, the U.S. Food and Drug Administration, or the FDA, or another regulatory authority were to require us to conduct clinical trials or other testing beyond those that we currently contemplate will be required for the completion of clinical development of ezutromid or ridinilazole or any other future product candidate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional resources and time on the completion of clinical development of that product candidate.

General and Administration Expenses

General and administration expenses consist primarily of salaries and benefits related to our executive, finance, business development, human resources and support functions. Other general and administration expenses include share-based compensation expenses, facility-related costs and expenses associated with the requirements of being a listed public company in the United Kingdom and the United States, including insurance, legal, professional, audit and taxation services fees.

We anticipate that our general and administration expenses will continue to increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our product candidates. We also anticipate continued increased accounting, audit, regulatory, compliance, insurance and investor and public relations expenses associated with being a publicly traded company in the United Kingdom and the United States. Our ADSs have traded on the NASDAQ Global Market since March 5, 2015.

Taxation

As a U.K. resident trading entity, we are subject to U.K. corporate taxation. Due to the nature of our business, we have generated losses since inception. To date, we have not recognized a deferred tax asset with respect to these tax losses because we do not consider it probable that there will be suitable taxable profits in the foreseeable future based on the evidence available. As a company that carries out extensive research and development activities, we benefit from the U.K. research and development tax credit regime and are able to surrender some of our trading losses that arise from our research and development activities for a cash rebate ranging from 8.80% to 33.35% of eligible research and development expenditure. In the event we generate revenues in the future, we may benefit from the “patent box” initiative that allows profits attributable to revenues from patents or patented

products to be taxed at a lower rate than other revenue. This relief applies to profits earned from April 1, 2013 and following the transitional arrangements that will phase in the relief, the rate of tax for relevant streams of revenue for companies receiving this relief will be 10%.

Change in Accounting Policy—Financial Liabilities

Following an IFRS Interpretations Committee agenda decision in May 2016 on the application of IAS 20, "*Accounting for Government Grants and Disclosure of Government Assistance,"* we changed our accounting policy regarding charitable funding arrangements with the Wellcome Trust and the U.S. not for profit organizations, the Muscular Dystrophy Association, or MDA, and Duchenne Partners Fund, or DPF, which has resulted in an adjustment to the comparative financial statements. In exchange for the funding provided, these arrangements require us to pay royalties on potential future revenues generated from these projects and also give the counterparties certain rights over the intellectual property if the compound is not exploited. The IFRS Interpretations Committee decision has clarified that such arrangements result in a financial liability. The estimate of each financial liability is initially recognized at fair value using a discounted cash flow model with the difference between the fair value of the liability and the cash received considered to represent a charitable grant. When determining the fair value on initial recognition, the significant assumptions in the model include the estimation of the timing and the probability of successful development leading to commercialization of the project related results and related estimates of future cash flows. Estimated future cash flows include expected sources of revenue (including commercial sales and upfront payments, milestone payments and royalties from potential licensing arrangements) and are calculated using estimated geographical market share and associated pricing. The financial liabilities are subsequently measured at amortized cost using a discounted cash flow model which calculates the risk adjusted net present values of estimated potential future cash flows for the respective projects related to the Wellcome Trust and U.S. not for profit organizations. The financial liabilities are re-measured when there is a specific significant event that provides evidence of a significant change in the probability of successful development such as the completion of a phase of research or changes in use or market for a product. The models will be updated for changes in the clinical probability of success and other associated assumptions with the discount rate remaining consistent within the model. Re-measurements of the financial liability are recognized in the income statement as finance costs. Grant income is recognized as other operating income in accordance with IAS 20, "*Accounting for Government Grants and Disclosure of Government Assistance*," at the same time as the underlying expenditure is incurred, provided that there is reasonable assurance that we will comply with the conditions. Amounts received from, and subsequent payments to, the corresponding counterparty in the funding agreement which relate to the financial liability will be presented within the financing activities section in the Consolidated Statement of Cash Flows.

This change in accounting policy has been reflected retrospectively in our audited consolidated financial statements included at the end of this Annual Report.

The impact of this change in accounting policy on the consolidated financial statements is a reduction in other income historically recognized, a change in the level of accrued income accounted for as grant income and the recognition of a financial liability and finance costs associated with the unwinding of the discount and remeasurment of the liability.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with IFRS as issued by the IASB. In the preparation of these financial statements, we are required to make judgments, estimates and assumptions about the carrying amounts of assets and liabilities that are not readily apparent from other sources. The estimates and associated assumptions are based on historical experience and other factors that are considered to be relevant. Actual results may differ from these estimates under different assumptions or conditions. Our estimates and assumptions are reviewed on an ongoing basis. Revisions to accounting estimates are recognized in the period in which the

estimate is revised if the revision affects only that period or in the period of the revisions and future periods if the revision affects both current and future periods.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing at the end of this Annual Report, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

Revenue Recognition

We recognize revenue from licensing fees, collaboration fees, development, regulatory and approval milestone fees, sales milestones and royalties. Agreements generally include a non-refundable up-front fee, milestone payments, the receipt of which is dependent upon the achievement of certain clinical, regulatory or commercial milestones, as well as royalties on product sales of licensed products, if and when such product sales occur. For these agreements, we are required to apply judgement in the allocation of total agreement consideration to the separately identifiable components on a reliable basis that reasonably reflects the selling prices that might be expected to be achieved in stand-alone transactions. We are required to make a judgement on those components which can be recognized immediately and those to which we apply the percentage of completion revenue recognition method.

Recognition of Research and Development Expenses

We recognize expenses incurred in carrying out our research and development activities in line with our best estimation of the stage of completion of each separately contracted study or activity. This includes the calculation of research and development accruals at each period to account for expenditure that has been incurred. This requires estimations of the full costs to complete each study or activity and also estimation of the current stage of completion. There have been no material adjustments to estimates based on the actual costs incurred for the periods presented. In all cases, we expense the full cost of each study or activity by the time the final study report or, where applicable, product, has been received.

We will recognize an internally generated intangible asset arising from our development activities only when an asset is created that can be identified, it is probable that the asset created will generate future economic benefits and the development cost of the asset can be measured reliably. We have determined that regulatory approval is the earliest point at which the probable threshold for the creation of an internally generated intangible asset can be achieved. We therefore expense all research and development expenditure incurred prior to achieving regulatory approval as it is incurred. None of our product candidates have yet received regulatory approval.

Financial liabilities on funding arrangements

When entering into funding agreements with charitable and not for profit organizations, we are required to assess whether, based on the terms of the agreement, we can avoid a transfer of cash only by settling a non-financial obligation. An example of this would be the obligation to transfer the rights to the research to a funding provider. In the circumstances where we cannot avoid the obligation, all or part of the funding agreement should be accounted for as a financial liability rather than as a charitable grant. In calculating the financial liabilities, both at inception and when it is subsequently re-measured, a number of assumptions need to be made by management which include significant estimates. Assumptions used in the model include the following: reported disease prevalence; expected market share based on management's estimates; drug reimbursement pricing in different territories, potential licensing terms which may be offered to us (for relevant products); expected patent life; and the timing and probabilities of achieving clinical development milestones which are based on industry standards and adjusted for therapy area. Discount factors in the range of 16% to 18% have been used in the model which has been calculated using appropriate measures and rates which could have been obtained in the period that the funding agreement was entered into. Sensitivity analysis is included in the notes to the financial statements and has been calculated based on a range of discount factors, estimated level of revenue and development

probabilities of success. The financial liabilities are re-measured, and we are required to apply judgement, when there is a specific significant event that provides evidence of a significant change in the probability of successful development such as the completion of a phase of research or changes in use or market for a product.

Share-based Compensation

We measure share options at fair value at their grant date in accordance with IFRS 2, "*Stock-based Payment*." We calculate the fair value of the share option using either the Black-Scholes model, or for options with performance conditions, a simulation model. We charge the fair value to the income statement over the expected vesting period. In the case of options that are issued below market value, the fair value will be higher than an option granted at market value, and we recognize a larger charge for such options in the income statement.

Results of Operations

Comparison of Years Ended January 31, 2017 and 2016

The amounts in the discussion below comparing results of operations for the years ended January 31, 2017 and 2016 have been adjusted following the change in accounting policy regarding charitable funding arrangements. See "—Change in Accounting Policy—Financial Liabilities."

The following table summarizes the results of our operations for the years ended January 31, 2017 and 2016, together with the changes to those items:

	Year ended January 31,		Change 2017 vs. 2016	
	2017	2016	Increase/(Decrease)	
	(in thousands, except percentages)			
Revenue	£ 2,304	£ —	£ 2,304	—
Other operating income	72	1,281	(1,209)	(94.4)%
Operating expenses				
Research and development	(18,952)	(16,856)	2,096	12.4
General and administration	(8,277)	(4,771)	3,506	73.5
Operating loss	(24,853)	(20,346)	4,507	22.2
Finance income	8	30	(22)	(73.3)
Finance cost	(862)	(2,879)	(2,017)	(70.1)
Loss before income tax	(25,707)	(23,195)	2,512	10.8
Income tax credit	4,336	3,058	1,278	41.8
Loss for the year	£(21,371)	£(20,137)	£ 1,234	6.1%

Revenue

Revenue of £2.3 million was recognized during the year ended January 31, 2017 following our entry into an exclusive license and collaboration agreement with Sarepta. Under the terms of the agreement, we received an upfront payment of $40.0 million (£32.8 million) from Sarepta. We have assessed the agreement, and we believe the development services to be indistinguishable and as a result the upfront payment has been initially reported as deferred income on the Consolidated Statement of Financial Position and is being recognized as revenue over the development period.

Other Operating Income

Other operating income decreased by £1.2 million, or 94.4%, to £0.07 million during the year ended January 31, 2017 from £1.3 million during the year ended January 31, 2016. Income attributed to the funding agreement with

the Wellcome Trust has been adjusted and is now been recognized in full with the completion of our CoDIFy Phase 2 clinical trial of ridinilazole. For more information, see "—Change in Accounting Policy—Financial Liabilities." Income recognized as part of the funding from Innovate UK for the DMD program decreased by £0.5 million to £0.06 million for the year ended January 31, 2017 from £0.6 million for the year ended January 31, 2016. The decrease in income is in line with the achievement of milestones under the funding agreement. Further, in September 2016, we elected to withdraw from the Innovate UK funding agreement in order to enable us to take advantage of more tax efficient opportunities related to research and development expenditure.

Operating Expenses

Research and Development Expenses

Research and development expenses increased by £2.1 million, or 12.4%, to £19.0 million for the year ended January 31, 2017 from £16.9 million for the year ended January 31, 2016. This increase was primarily due to increased spending related to our DMD program. During the year ended January 31, 2017, expenses related to our DMD program increased by £2.0 million to £9.5 million from £7.5 million for the year ended January 31, 2016. This increase included £1.9 million related to our ezutromid clinical activities, £0.9 million associated with manufacturing costs for our clinical trials, and was offset by a decrease of £0.8 million related to research associated with our second generation utrophin modulator program, the development of which was put on hold in September 2016 due to the substantial increase in ezutromid plasma levels achieved with our F6 formulation of ezutromid. During the year ended January 31, 2017, expenses related to our CDI program decreased by £1.5 million to £4.1 million from £5.6 million for the year ended January 31, 2016. This decrease was primarily due to the completion of our CoDIFy Phase 2 clinical trial of ridinilazole. During the year ended January 31, 2017, other research and development expenses increased by £1.6 million to £5.4 million from £3.8 million for the year ended January 31, 2016. This increase was due to a £1.3 million increase in staffing costs, a £0.1 million increase in internal regulatory costs and a £0.1 million increase in the share-based payment charge allocated to research and development expenses, which resulted from increased employee headcount within our DMD and CDI program teams.

General and Administration Expenses

General and administration expenses increased by £3.5 million, or 73.5%, to £8.3 million for the year ended January 31, 2017 from £4.8 million for the year ended January 31, 2016. This increase was due to a £1.5 million increase in legal and professional expenses, an increase of £0.7 million in staff related costs, an increase of £0.2 million in share based payment expense and an increase of £0.1 million in overhead and facility related costs as well as a net negative movement of £1.0 million in exchange rate variance.

Finance Income

Finance income decreased by £0.02 million to £0.01 million for the year ended January 31, 2017 from £0.03 million for the year ended January 31, 2016. This decrease was due to reduced bank interest received on cash deposits as a result of a lower available bank interest rate.

Finance Cost

Following an IFRS Interpretations Committee agenda decision in May 2016 on the application of IAS 20, "*Accounting for Government Grants and Disclosure of Government Assistance*," we changed our accounting policy regarding charitable funding arrangements from the Wellcome Trust and U.S. not for profit organizations. For more information, see "—Change in Accounting Policy—Financial Liabilities." Finance costs relate to the subsequent re-measurement of the financial liability recognized in respect of funding arrangements and the unwinding of the discounts associated with the liabilities. Finance costs decreased by £2.0 million, or 70.1%, to

£0.9 million for the year ended January 31, 2017 from £2.9 million for the year ended January 31, 2016 as there was not a subsequent re-measurement of the financial liability during the year ended January 31, 2017, with finance costs relating to the unwinding of the discount only. During the year ended January 31, 2016, of the total finance cost of £2.9 million, £2.6 million related to the re-measurement of the financial liability following positive data in the DMD and CDI clinical programs that increased the probabilities of success.

Income Tax Credit

Our income tax credit increased by £1.3 million, or 41.8%, to £4.3 million for the year ended January 31, 2017 from £3.0 million for the year ended January 31, 2016. This increase was the result of our increased expenditure on research and development and a related increase in our research and development tax credit.

Comparison of Years Ended January 31, 2016 and 2015

The amounts in the discussion below comparing results of operations for the years ended January 31, 2016 and 2015 have been adjusted following the change in accounting policy regarding charitable funding arrangements. See "—Change in Accounting Policy—Financial Liabilities."

The following table summarizes the results of our operations for the years ended January 31, 2016 and 2015, together with the changes to those items:

	Year ended January 31,		Change 2016 vs. 2015	
	2016	2015	Increase/(Decrease)	
	(in thousands, except percentages)			
Other operating income	£ 1,281	£ 1,888	£ (607)	(32.2)%
Operating expenses				
Research and development	(16,856)	(10,417)	6,439	61.8
General and administration	(4,771)	(3,704)	1,067	28.8
Operating loss	(20,346)	(12,233)	8,113	66.3
Finance income	30	51	(21)	(41.2)
Finance cost	(2,879)	(499)	2,380	477.0
Loss before income tax	(23,195)	(12,681)	10,514	82.9
Income tax credit	3,058	1,297	1,761	135.8
Loss for the year	£(20,137)	£(11,384)	£ 8,753	76.9%

Other Operating Income

Other operating income decreased by £0.6 million, or 32.2%, to £1.3 million during the year ended January 31, 2016 from £1.9 million during the year ended January 31, 2015. Income recognized as part of the Wellcome Trust Translational Award decreased by £0.3 million to £0.6 million for the year ended January 31, 2016 from £0.9 million for the year ended January 31, 2015 as a result of a lower contribution rate ascribed to Phase 2 activities as compared to Phase 1 activities under the terms of the funding agreement. As of January 31, 2016, we had an accrued income balance of £0.06 million related to cash due to be received from the Wellcome Trust, which we expected to receive, and did receive, within the following twelve months upon achievement of the final milestone of the Wellcome Trust Award when we delivered final reports related to the grant. All amounts in respect of the Wellcome Trust Translational Award have been adjusted following the change in accounting policy as more fully described above. Income recognized as part of the funding from Innovate UK for the DMD program decreased by £0.3 million to £0.6 million for the year ended January 31, 2016 from £0.9 million for the year ended January 31, 2015. The decrease in income was in line with the achievement of milestones during such period under the funding agreement.

Operating Expenses

Research and Development Expenses

Research and development expenses increased by £6.4 million, or 61.8%, to £16.8 million for the year ended January 31, 2016 from £10.4 million for the year ended January 31, 2015. This increase was primarily due to increased spending related to our DMD and CDI programs. During the year ended January 31, 2016, expenses related to our DMD program increased by £2.8 million to £7.5 million from £4.7 million for the year ended January 31, 2015. This increase included £1.6 million related to our ezutromid clinical activities, £1.2 million related to research associated with our second generation utrophin modulator program and our strategic alliance with the University of Oxford, £0.3 million associated with manufacturing costs for our clinical trials, and offset by a decrease of £0.3 million related to long term toxicity studies which were performed in 2014. During the year ended January 31, 2016, expenses related to our CDI program increased by £2.3 million to £5.5 million from £3.2 million for the year ended January 31, 2015. This increase was primarily due to costs incurred related to our then-ongoing Phase 2 clinical trials, as well as preparatory Phase 3 clinical trial costs. During the year ended January 31, 2016, other research and development expenses increased by £1.3 million to £3.8 million from £2.5 million for the year ended January 31, 2015. This increase was due to a £1.1 million increase in staffing costs, a £0.1 million increase in internal regulatory costs and a £0.1 million increase in the share-based payment charge allocated to research and development expenses, which resulted from increased employee headcount within our DMD and CDI program teams.

General and Administration Expenses

General and administration expenses increased by £1.0 million, or 28.8%, to £4.7 million for the year ended January 31, 2016 from £3.7 million for the year ended January 31, 2015. This increase was due to an £0.7 million increase in legal and professional expenses and other costs associated with being a publicly traded company in the United States as well as in the United Kingdom, an increase of £0.4 million in staff related costs, an increase of £0.2 million in overhead and facility related costs and an increase of £0.1 million in share based payment expense offset by £0.4 million recognized as a favorable exchange rate variance.

Finance Income

Finance income decreased by £0.02 million to £0.03 million for the year ended January 31, 2016 from £0.05 million for the year ended January 31, 2015. This decrease was due to reduced bank interest received on cash deposits as a result of a lower available bank interest rate.

Finance Cost

Following an IFRS Interpretations Committee agenda decision in May 2016 on the application of IAS 20, "*Accounting for Government Grants and Disclosure of Government Assistance*," we changed our accounting policy regarding charitable funding arrangements from the Wellcome Trust and U.S. not for profit organizations. For more information, see "—Change in Accounting Policy—Financial Liabilities." Finance costs relate to the subsequent re-measurement of the financial liability recognized in respect of income arrangements and the unwinding of the discounts associated with the liabilities. Finance cost increased by £2.4 million to £2.9 million for the year ended January 31, 2016 from £0.5 million for the year ended January 31, 2015. This increase was primarily driven by the re-measurement of the financial liabilities on funding arrangements following significant successful events in the associated clinical programs during the year ended January 31, 2016.

Income Tax Credit

Our income tax credit increased by £1.8 million, or 135.8%, to £3.1 million for the year ended January 31, 2016 from £1.3 million for the year ended January 31, 2015. This increase was the result of our increased expenditure on research and development and a related increase in our research and development tax credit.

Liquidity and Capital Resources

Sources of liquidity

Since our inception, we have incurred significant operating losses. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administration expenses will continue to increase in connection with conducting clinical trials for our lead product candidates, ezutromid for the treatment of DMD and ridinilazole for the treatment of CDI, and seeking marketing approval for ezutromid in the United States and other territories where we retain commercialization rights and ridinilazole in the United States and the European Union as well as other geographies. As a result, we will need additional capital to fund our operations, which we may obtain from additional financings, research funding, collaborations, contract and grant revenue or other sources. Additional capital, when needed, may not be available to us on acceptable terms, or at all.

To date, we have financed our operations primarily through issuances of our ordinary shares and American Depositary Shares, or ADSs, payments to us under our license and collaboration agreement with Sarepta and development funding and other assistance from government entities, philanthropic, non-government and not for profit organizations and patient advocacy groups for our product candidates. In particular, we have received funding from the Wellcome Trust, Innovate UK, Joining Jack, the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, Charley's Fund, Cure Duchenne, Foundation to Eradicate Duchenne and the Nash Avery Foundation.

As of January 31, 2017, we had cash and cash equivalents of £28.1 million.

In March 2014, we received net proceeds of £20.5 million from the issuance and sale of 16,923,077 ordinary shares in a private placement outside the United States. In March 2015, in our initial public offering in the United States, we received net proceeds of $32.7 million (£21.9 million) from the issuance and sale of 3,967,500 American Depositary Shares which represent 19,837,500 ordinary shares. In October 2016, in connection with our entry into an exclusive license and collaboration agreement with Sarepta, we received an up-front payment of $40.0 million (£32.8 million) from Sarepta.

Cash Flows

The following table summarizes the results of our cash flows for the years ended January 31, 2017, 2016 and 2015.

	Year ended January 31,			
	2017	2017	2016	2015
		(in thousands)		
Net cash inflow / (outflow) from operating activities	$15,280	£12,141	£(17,182)	£(11,009)
Net cash (outflow) / inflow from investing activities	(101)	(80)	(36)	16
Net cash inflow from financing activities	520	413	22,136	20,228
Net increase/(decrease) in cash and cash equivalents	$15,699	£12,474	£ 4,918	£ 9,235

Operating Activities

For the year ended January 31, 2017, we generated £12.1 million in cash from operating activities. This compares to net cash used in operating activities of £17.2 million for the year ended January 31, 2016. This net movement of £29.3 million was driven by our receipt of a $40.0 million (£32.8 million) upfront payment from Sarepta as part of our exclusive license and collaboration agreement with them. This was offset by an increase in research and development expenditure and general and administrative expenditure during the year ended January 31, 2017. There was also a £1.6 million increase in the amount of research and development tax credit received

during the year ended January 31, 2017 which was £3.0 million compared to £1.4 million received during the year ended January 31, 2016.

Net cash outflow from operating activities increased by £6.2 million, or 56.0%, to £17.2 million for the year ended January 31, 2016 compared to £11.0 million for the year ended January 31, 2015. This increase was driven by an increase in operating expenses and working capital requirements, offset by the receipt of a research and development tax credit that increased by £0.7 million, or 112.9%, to £1.4 million for the year ended January 31, 2016 compared to £0.7 million for the year ended January 31, 2015.

Investing Activities

Net cash outflows for the years ended January 31, 2017 and January 31, 2016 and net cash inflows for the year ended January 31, 2015 include the net amount of bank interest received on cash deposits less amounts paid to acquire property and equipment.

Financing Activities

Net cash inflow from financing activities for the year ended January 31, 2017 relates to proceeds from the exercise of warrants and the exercise of share options. Net cash inflow from financing activities for the years ended January 31, 2016 and 2015 relates to the proceeds received from the various sales of our equity securities, net of expenses. We received £22.1 million from the sale of equity securities during the year ended January 31, 2016 and £20.5 million from the sale of equity securities during the year ended January 31, 2015.

Funding Requirements

We anticipate that our expenses will increase substantially in connection with conducting clinical trials for our lead product candidates, ezutromid for the treatment of patients with DMD and ridinilazole for the treatment of patients with CDI, and seeking marketing approval for ezutromid in the United States and other territories where we retain commercialization rights and ridinilazole in the United States and the European Union as well as in other geographies. In addition, if we obtain marketing approval of ezutromid or ridinilazole, we expect to incur significant sales, marketing, distribution and outsourced manufacturing expense, as well as ongoing research and development expenses.

In addition, our expenses will increase if and as we:

- continue the research and development of the F3 formulation of ezutromid, the F6 formulation of ezutromid and future generation utrophin modulators that we are developing in collaboration with the University of Oxford and Sarepta;
- seek to identify and develop additional future product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical development;
- ultimately establish a sales, marketing and distribution infrastructure in jurisdictions where we have retained commercialization rights and scale up external manufacturing capabilities to commercialize any product candidates for which we receive marketing approval;
- acquire or in-license other product candidates and technology;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- expand our physical presence; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

We believe that our existing cash and cash equivalents, including an anticipated $22.0 million payment for a near-term development milestone under the license and collaboration agreement with Sarepta, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements through December 31, 2018. In our DMD program, while we anticipate that these capital resources will allow us to obtain top-line data for our Phase 2 clinical trial of ezutromid, which we refer to as PhaseOut DMD, we do not expect these capital resources will be sufficient to complete our planned randomized, placebo controlled clinical trial of ezutromid. In addition, in our CDI program, while we also anticipate that these capital resources will allow us to conduct activities to prepare ridinilazole for our two, planned Phase 3 clinical trials, we do not expect to be able to complete these trials without significant additional capital.

We have based the foregoing estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. This estimate assumes, among other things, that we do not obtain any additional funding through grants and clinical trial support or through new collaboration arrangements. Our future capital requirements will depend on many factors, including:

- the progress, costs and results of clinical trials of ezutromid for DMD and ridinilazole for CDI;
- the scope, progress, costs and results of preclinical development, laboratory testing and clinical trials for our F3 formulation of ezutromid, the F6 formulation of ezutromid and future generation utrophin modulators that we are developing in collaboration with the University of Oxford and Sarepta;
- the number and development requirements of other future product candidates that we pursue;
- the costs, timing and outcome of regulatory review of ezutromid, ridinilazole and our other future product candidates;
- the costs and timing of commercialization activities, including product sales, marketing, distribution and manufacturing, for any of our product candidates that receive marketing approval;
- subject to receipt of marketing approval, revenue received from commercial sales of ezutromid, ridinilazole or any of our other future product candidates;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against any intellectual property-related claims;
- the amounts we receive from Sarepta for development, regulatory and sales milestones and royalty payments under our license and collaboration agreement;
- our ability to establish and maintain collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the extent to which we acquire or invest in other businesses, products and technologies;
- the rate of the expansion of our physical presence; and
- the costs of operating as a public company in the United States in addition to in the United Kingdom.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, collaborations, strategic alliances, grants and clinical trial support from governmental entities and philanthropic, non-government and not for profit organizations and patient advocacy groups, debt financings, and marketing, distribution or licensing arrangements. We do not have any committed external source of funds other than amounts we may receive from Sarepta for potential option exercise, development, regulatory and sales milestones and royalties payments under our license and collaboration agreement. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing shareholders, including our ADS holders, will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing shareholders and ADS holders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends or other distributions. If we raise additional funds through

collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Tabular Disclosure of Contractual Obligations

The following table summarizes our contractual commitments and obligations as of January 31, 2017.

	Payments due by period				
	Total	Less than 1 Year	Between 1 and 3 Years	Between 3 and 5 Years	More than 5 Years
			(in thousands)		
Operating lease obligations	£ 210	£ 88	£ 122	£ —	—
Contractual obligations	2,283	830	1,453	—	—
Total contractual cash obligations	£2,493	£ 918	£ 1,575	£ —	—

We enter into operating leases in the normal course of our business. On February 17, 2017, we entered into a lease for new office space in Abingdon, U.K. The term of the lease is ten years, and we are required to pay annual rent under the lease of £32,888 for the first year, £132,908 for the second year and £166,135 for each of the third, fourth and fifth years of the lease. Following the fifth year of the lease, we may terminate the lease for a break fee payment of £55,378 or be obligated under the lease for the remaining five-year term at the then-market rate for the leased property.

The contractual obligations in the preceding table reflect our obligation to fund a drug research and discovery program in the University of Oxford laboratories that is described in more detail under "University of Oxford" below.

Under various agreements, including those described below, we will be required to pay royalties and make milestone payments to third parties. See "Business —Our Collaborations and Funding Arrangements" in this Annual Report for additional information regarding these agreements. The preceding table excludes contingent payment obligations, such as royalties and milestones, which are described in more detail below, because the amount, timing and likelihood of payment are not known.

University of Oxford

In November 2013, we acquired all of the outstanding equity of MuOx Limited, or MuOx, a spin out of the University of Oxford. In connection with that acquisition, we and MuOx entered into a set of agreements with the University of Oxford and its technology transfer division, Isis Innovation Limited, or Isis, which is now known as Oxford University Innovation Limited. Under the research sponsorship agreement that we entered into with the University of Oxford and Isis in November 2013, amended and restated in July 2014 and amended in November 2015, we have agreed to fund a drug research and discovery program in the University of Oxford laboratories to identify and research utrophin modulators to treat DMD. The University of Oxford is responsible for conducting this program. Isis has no obligations under the research sponsorship agreement. We have agreed to fund up to £4.3 million for this purpose over the initial six-year research period ending in November 2019. If we exercise our right to extend the research period by an additional year, we have agreed to fund an additional £0.8 million, for a total of £5.2 million. As of January 31, 2017, we had paid to the University of Oxford £2.1 million of this amount. Under an option agreement that we, the University of Oxford and Isis entered into in November 2013, which we amended in November 2015, which we refer to as the option agreement, Isis granted us an exclusive option to license certain intellectual property, or IP, arising under the research sponsorship

agreement and certain other IP arising from research and development at the University of Oxford, which we refer to as arising IP. We paid Isis £10,000 in connection with entering into the amendment to the option agreement. If we exercise our option to obtain a license under arising IP, we would be obligated to pay Isis a specified sum in option exercise fees. For any arising IP for which we have exercised the option and that comprises new chemical entities or compounds, which we refer to as optioned compounds, we would obtain an exclusive, sublicensable license. We are obligated to pay milestone payments of up to £75,000 upon the achievement of specified development milestones, whether such milestones occur prior to or after our exercise of the option to obtain an exclusive sublicensable license. Following exercise of such an option we would also be obligated to pay milestone payments upon the achievement of specified regulatory milestones with respect to each optioned compound. The specified regulatory milestone payment is due each time the specified regulatory milestone is achieved with respect to an optioned compound and, if each optioned compound achieved each regulatory milestone once, we would be obligated to pay Isis a total of £3.7 million in regulatory milestone payments for each optioned compound.

We would also be obligated to pay Isis a low single digit royalty of net sales by us, our affiliates or sublicensees of any product containing an optioned compound or on any payments we receive in connection with granting a sublicense under the licensed arising IP. For any arising IP for which we have exercised the option and that does not comprise new chemical entities or compounds, we would obtain an exclusive license, which we could sublicense with Isis' prior written consent. We and Isis would negotiate the milestone payments and any other payments that we would be obligated to pay to Isis with respect to such IP.

Wellcome Trust

In October 2012, we entered into a translation award funding agreement with The Wellcome Trust Limited, as trustee of the Wellcome Trust, to support a Phase 1 and a Phase 2 clinical trial of ridinilazole for the treatment of CDI. We refer to the translation award funding agreement as the translation award agreement. We refer to any compound or product that is covered by IP rights created under the translation award agreement or our prior agreement with the Wellcome Trust, or that is covered by IP rights to which we had rights prior to October 2009 and that relate to the activities under our agreements with the Wellcome Trust, as the award products. Under the terms of a related revenue sharing agreement that we would enter into with the Wellcome Trust to permit our development and commercialization of the award products, the Wellcome Trust is entitled to receive a tiered portion of the net revenue, ranging from a mid-single digit percentage up to a mid-twenties percentage, that we or our affiliates receive from our development and commercialization of the award products and the related IP. In addition, we would be obligated to pay the Wellcome Trust a milestone in a specified amount if cumulative net revenue exceeds a specified amount.

University College London

On March 23, 2010, we entered into a collaborative research agreement with the School of Pharmacy, University of London which was later novated on November 28, 2011 by the School of Pharmacy to University College London. As part of this agreement, and in consideration of their role in the development of the initial compound series from which ridinilazole was later identified, we agreed to pay the School of Pharmacy (now University College London) a low single-digit share of all revenue received by us in respect of ridinilazole, including any pre-commercial licensing revenue, up to a maximum of £1.0 million.

U.S. Not for Profit Organizations

Muscular Dystrophy Association

In December 2011, we entered into a grant agreement with the Muscular Dystrophy Association, Inc., or MDA, to partially fund a Phase 1 clinical trial of ezutromid to treat DMD. Under the terms of the grant agreement, we have agreed to make specified milestone payments to MDA during our or our affiliates' development and

commercialization of pharmaceutical products containing the small molecules that can upregulate the utrophin gene, including ezutromid and compounds to which we have rights, which we refer to as the project products. Because we raised more than a specified aggregate amount of funding, we paid a specified sum to MDA under the terms of the grant agreement, which we refer to as the MDA cash infusion milestone payment. We have also agreed to pay MDA a specified lump sum amount, less any previously paid MDA cash infusion milestone payment, following the regulatory approval of any project product for use or sale in the United States or European Union for the treatment of DMD or Becker muscular dystrophy, or BMD, and an additional specified sum upon achievement of a commercial milestone. We would be obligated to pay MDA a low single digit percentage royalty of worldwide net sales by us, our affiliates or licensees of any project product. If we assign our rights to any of the compounds subject to the grant agreement or experience specified change in control events, MDA may require our assignee to assume our obligations under the MDA grant agreement with respect to the assigned rights, or require us to pay MDA the greater of a low single digit percentage of the fair market value of the assigned rights, or an amount that would give MDA an internal rate of return of a low double digit percentage on its grant to us.

Duchenne Partners Fund

In December 2011, we entered into a grant agreement with the Duchenne Partners Fund, LLC, or DPF, to partially fund a Phase 1 clinical trial of ezutromid to treat DMD. Under the DPF grant agreement, we have agreed to make specified milestone payments to DPF during our or our affiliates' development and commercialization of pharmaceutical products containing the small molecules that can upregulate the utrophin gene, including ezutromid and compounds with similar mechanisms of action to which we have rights, which we refer to as project products. Because we raised more than a specified aggregate amount of funding, we paid a specified sum to DPF under the terms of the agreement, which we refer to as the DPF cash infusion milestone payment.

We have also agreed to pay DPF a specified lump sum amount, less any previously paid DPF cash infusion milestone payment, following the regulatory approval of any project product for use or sale in the United States or European Union for the treatment of DMD or BMD and an additional specified sum upon achievement of a commercial milestone. We would be obligated to pay DPF a low single digit percentage royalty of worldwide net sales by us, our affiliates or licensees of any project product. If we assign our rights to any of the compounds subject to the DPF grant agreement or experience specified change in control events, DPF may require our assignee to assume our obligations under the DPF grant agreement with respect to the assigned rights, or require us to pay DPF the greater of a low single digit percentage of the fair market value of the assigned rights, or an amount that would give DPF an internal rate of return of a low double digit percentage on its grant to us.

The total amount payable with respect to regulatory milestones under the U.S. not for profit organization agreements would be $2.5 million if we meet all regulatory milestones.

Other Contracts

In addition, we enter into contracts in the normal course of business with contract research organizations to assist in the performance of our research and development activities and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancellable contracts and not included in the table of contractual obligations and commitments.

Off-Balance Sheet Arrangements

We do not have any, and during the periods presented we did not have any, off-balance sheet arrangements, other than the contractual obligations and commitments described above.

Jumpstart Our Business Startups Act of 2012

As a company with less than $1 billion in revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart our Business Startups Act of 2012, or the JOBS Act. An emerging growth company may take advantage of specified reduced reporting and other burdens that are otherwise applicable generally to public companies. These provisions include:

- an exemption from compliance with the auditor attestation requirement on the effectiveness of our internal controls over financial reporting;
- an exemption from compliance with any requirement that the Public Company Accounting Oversight Board may adopt regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure about the company's executive compensation arrangements; and
- exemptions from the requirements to obtain a non-binding advisory vote on executive compensation or a shareholder approval of any golden parachute arrangements.

We may take advantage of these provisions until January 31, 2021 or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company if we have more than $1 billion in annual revenues, have more than $700 million in market value of our share capital held by non-affiliates or issue more than $1 billion of non-convertible debt over a three-year period. We may choose to take advantage of some, but not all, of the available benefits under the JOBS Act. We have taken advantage of some reduced reporting burdens in this Annual Report. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This provision allows an emerging growth company to delay the adoption of some accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of delayed adoption of new or revised accounting standards and, therefore, we will be subject to the same requirements to adopt new or revised accounting standards as other public companies that are not emerging growth companies.

Item 6: Directors, Senior Management and Employees

A. Directors and Senior Management

The following table sets forth the names, ages and positions of our executive officers, key employees and directors as of the date of this Annual Report:

Name	Age	Position
Executive Officers		
Glyn Edwards	61	Chief Executive Officer, Executive Director
Erik Ostrowski	44	Chief Financial Officer
David Roblin*	50	Chief Operating Officer and President of Research and Development
Key Employees		
Ralf Rosskamp	64	Chief Medical Officer
Jonathon Tinsley	58	Chief Scientific Officer, DMD
Richard Vickers	41	Chief Scientific Officer, Antimicrobials
Non-Employee Directors		
Frank Armstrong(2)(3)(4)	60	Non-Executive Chairman
Barry Price(1)(3)(4)	73	Non-Executive Director
Stephen Davies(2)(3)(4)	67	Non-Executive Director
Leopoldo Zambeletti(3)(4)	48	Non-Executive Director
Valerie Andrews(1)(2)(3)(4)	57	Non-Executive Director
David Wurzer(1)(3)(4)	58	Non-Executive Director

(1) Member of the Audit Committee.
(2) Member of the Remuneration Committee.
(3) Member of the Nominating and Corporate Governance Committee.
(4) An "independent director" as such term is defined in Rule 10A-3 under the Exchange Act.
* David Roblin will take up his new role as Chief Operating Officer and President of Research and Development on an interim basis in April 2017, becoming full-time in June 2017.

Executive Officers

Glyn Edwards has served as our Chief Executive Officer and a member of our board of directors since April 2012. Prior to joining our company, Mr. Edwards served as interim Chief Executive Officer of the BioIndustry Association, a U.K. trade organization, from November 2011 to June 2012, and Chief Executive Officer at Antisoma plc, a publicly traded biotechnology company specializing in the development of novel drugs for the treatment of cancer, from 1998 to 2011. Mr. Edwards also previously served as Vice President of Business Development at Therapeutic Antibodies Ltd. Mr. Edwards received a BSc in Biochemistry from Bristol University and a MSc in Economics from the London Business School. We believe that Mr. Edwards is qualified to serve as a member of our board of directors because of his extensive executive leadership and business development experiences in the life sciences industry.

Erik Ostrowski has served as our Chief Financial Officer since June 2014. Prior to joining our company, Mr. Ostrowski served as Vice President of Finance at Organogenesis Inc., a biotechnology company, from 2010 to 2014. Prior to that, Mr. Ostrowski worked in investment banking, most recently as a Director with Leerink Partners LLC. Mr. Ostrowski began his career as an accountant with Coopers & Lybrand. Mr. Ostrowski received a BS in Accounting and Economics from Babson College and an MBA from the University of Chicago Booth School of Business.

David Roblin will serve as our Chief Operating Officer and President of Research and Development in a part-time capacity beginning in April 2017 and become full-time in June 2017. Dr. Roblin has been acting as a

research and development adviser to us since 2014. Dr. Roblin has served as the Chief Operating Officer and Director of Scientific Translation at the Francis Crick Institute in London from 2014 to 2017. Prior to that, Dr. Roblin was Head of Research, Site Director and Chief Medical Officer for Europe R&D at Pfizer Inc. from 2008 to 2011 and was Head of Therapy Area for Anti-infectives at Bayer AG from 1997 to 1999. After Dr. Roblin left Pfizer Inc., he was Chief Medical Officer to a number of biotechnology companies including Creabilis SA where he held that role from 2011 until 2014. Dr Roblin has a degree in biochemistry from University College London and later qualified in medicine from St George's Hospital. He is a Fellow of the Royal College of Physicians and a Fellow of the Faculty of Pharmaceutical Medicine. He is an honorary Professor of Medicine at Swansea University and Professor of Translational Medicine at St George's. He is also a member of the board of directors of MedCity and Destiny Pharma Ltd. Before entering the life sciences industry, Dr Roblin practiced medicine for five years.

Key Employees

Ralf Rosskamp has served as our Chief Medical Officer since September 2015. Prior to joining our company, Dr. Rosskamp served as Vice President, Global Clinical Development at NPS Pharmaceuticals, Inc. from July 2013 to May 2015, which was subsequently acquired by Shire Pharmaceuticals. Prior to that, Dr. Rosskamp was Executive Vice President, Research and Development, at Ikaria, Inc., a biotherapeutics company from 2007 to 2010. Dr. Rosskamp has also served as Executive Vice President, R&D of Kos Pharmaceuticals, Inc. (acquired by Abbott Laboratories), Vice President, Global Therapeutic Area Endocrinology, Metabolism, Rheumatology and Bone at Aventis Pharmaceuticals Inc. and Head, Clinical Research—Metabolism and Global Clinical Director, Recombinant Products at Hoechst AG. In his academic career, Dr. Rosskamp served as Associate Professor, Pediatric Endocrinology in the Department of Pediatrics at the University of Bonn. He received an M.D. from the University of Bonn and is a board-certified pediatrician in Germany.

Jonathon Tinsley joined our company in April 2005 and has served as our Chief Scientific Officer, DMD, since October 2013. During his time with our company, Dr. Tinsley has overseen the development of our utrophin modulation program for the treatment of DMD from early discovery through to patient clinical trials. Dr. Tinsley previously worked in the laboratories of Professor Kay Davies at the University of Oxford. He is the co-author of over 35 peer reviewed scientific publications related to utrophin biology and the co-inventor on a number of patents related to utrophin biology. Prior to joining our company, Dr. Tinsley was the Head of Biology at Oxagen Limited and a Senior Research Fellow at the Medical Research Council. Dr. Tinsley received a Ph.D. in cancer studies from the University of Birmingham and a B.Sc. in microbiology from the University of Leeds.

Richard Vickers joined our company in 2003 and has served as our Chief Scientific Officer, Antimicrobials, since October 2013. During his time at our company, Dr. Vickers has worked in a variety of roles involved in the development and management of various antibacterial therapeutic programs, including our antibiotic program for the treatment of CDI. Prior to joining our company, Dr. Vickers undertook postdoctoral research studies with Professor Stephen Davies at the University of Oxford and held a Stipendiary Lectureship in organic chemistry at St. Catherine's College in Oxford. Dr. Vickers received a Ph.D. in organic chemistry from the University of Reading and a B.Sc. in chemistry from King's College London.

Non-Employee Directors

Frank Armstrong has served as a member of our board of directors since November 2012 and Non-Executive Chairman since June 2013. Dr. Armstrong is currently President of Dr Frank M Armstrong Consulting Limited, a position he has held since 2012. Prior to this, Dr. Armstrong led Medical Science and Innovation at Merck Serono, the biopharmaceutical division of Merck KGaA, from 2010 to 2011. Dr. Armstrong was also Head of Worldwide Product Development at Bayer AG from 1998 to 2001 and held various positions at ICI plc and Zeneca plc, now AstraZeneca plc, from 1985 to 1998. Dr. Armstrong has served as the Chief Executive Officer at five biotechnology companies, including Fulcrum Pharma plc, which is listed on AIM, CuraGen Corporation, a NASDAQ-listed company that was acquired by Celldex Therapeutics, Inc., Bioaccelerate Holdings Inc., Provensis Ltd. and Phoqus Pharmaceuticals plc. Dr. Armstrong is the Non-Executive Chairman of Faron

Pharmaceuticals Oy and Caldan Therapeutics Ltd; a Non-Executive Director of Juniper Pharmaceuticals Inc. (formerly Columbia Laboratories Inc.), which is listed on NASDAQ, and Mereo Biopharma Group plc; and a Member of the Strategic Advisory Board of HealthCare Royalty Partners and Epidarex Capital. Dr. Armstrong received an honors degree in Biochemistry and an MBChB(MD) in Medicine from the University of Edinburgh in Scotland. He is a Fellow of the Royal College of Physicians, Edinburgh and a Fellow of the Faculty of Pharmaceutical Physicians. We believe that Dr. Armstrong is qualified to serve on our board of directors because of his extensive experience in the biotechnology industry and his medical background.

Barry Price has served as a member of our board of directors since September 2006. Dr. Price spent 28 years with the Glaxo Group of companies, where he held several executive positions including, Managing Director of Glaxochem Ltd. from 1993 to 1995 and Research Director of Glaxo Group Research from 1989 to 1993. Dr. Price also served as a Non-Executive Director of Shire plc, a biopharmaceutical company that is listed on the London Stock Exchange and NASDAQ, from 1996 to 2009, during which time he was involved in developing the company into one of the U.K.'s largest life sciences companies. Dr. Price has previously held directorships at Chiroscience plc, Celltech Group plc, Pharmagene plc, Antisoma plc, and BioWisdom Ltd. Dr. Price received a BSc in chemistry and a Ph. D. in chemistry from the University of Sheffield, and he is a Fellow of the Royal Society of Chemistry. We believe Dr. Price is qualified to serve on our board of directors because of his extensive experience in the pharmaceutical and life sciences industries.

Stephen Davies has served as a member of our board of directors since November 2013 and previously served as a member of our board of directors from 2004 to February 2013. Professor Davies has been a professor at the University of Oxford since 1996 and was appointed Waynflete Professor of Organic Chemistry and Fellow of Magdalen College in 2006. Professor Davies' areas of expertise include medicinal and asymmetric chemistry. He has published extensively and received numerous awards in his field. Professor Davies co-founded our company, as well as other University of Oxford spin-out companies. He was the founder and Non-Executive Chairman of MuOx Limited and OxRay Ltd and Non-Executive Chairman of Scientific Research Capital Ltd.; is a founder and Non-Executive Director of the Oxstem group of companies; and is a Non-Executive Director of Oxford University Innovation Ltd. Professor Davies received a BA in Chemistry from the University of Oxford, a D.Phil in Organic Chemistry from the University of Oxford, and a D.Sc. in Organic Chemistry from the University of Paris. We believe Professor Davies is qualified to serve on our board of directors because of his extensive experience as an academic and entrepreneur in the biopharmaceutical industry.

Leopoldo Zambeletti has served as a member of our board of directors since May 2014. Mr. Zambeletti has served as an independent strategic advisor to life sciences companies since 2013, focusing on mergers and acquisitions, out-licensing deals and financing strategy. Prior to this, Mr. Zambeletti worked in investment banking for 19 years, during which time he led the European Healthcare Investment teams at JP Morgan and at Credit Suisse. He is a Non-Executive Director of Nogra Pharma Ltd., Advanced Accelerator Applications, Faron Pharmaceuticals Oy, Dignity Services Ltd. and an advisor and co-founder to the U.S. medtech company Qardio. Mr. Zambeletti began his career as an accountant at KPMG. He received his degree in Business Administration from Bocconi University. We believe Mr. Zambeletti is qualified to serve on our board of directors because of his extensive experience in the finance and life sciences industries.

Valerie Andrews has served as a member of our board of directors since September 2014. Most recently, Ms. Andrews served from May 2011 until May 2014 as General Counsel at Vertex Pharmaceuticals Incorporated, a biopharmaceutical company focused on small molecule therapies for cystic fibrosis and other indications. From 2002 to May 2011, Ms. Andrews served in various legal roles at Vertex, including as Deputy General Counsel and Chief Compliance Officer. Prior to joining Vertex, Ms. Andrews was the Executive Director of Licensing for Massachusetts General Hospital and Brigham and Women's Hospital from September 2001 to March 2002. From 1989 to 2001, Ms. Andrews served as a corporate lawyer at Hill & Barlow PC, where she became a partner in 1997. In her professional roles, Ms. Andrews has garnered expertise in areas including corporate strategy, strategic transactions, corporate governance, executive compensation, risk management, and compliance. Ms. Andrews served as a Non-Executive Director of Juniper Pharmaceuticals Inc. (formerly

Columbia Laboratories) from 2005 until 2015. Ms. Andrews received a B.A. in Chemistry and Psychology from Duke University and a J.D. from Boston College. We believe Ms. Andrews is qualified to serve on our board of directors because of her extensive skills in business and legal matters related to the pharmaceuticals industry.

David M. Wurzer has served as a member of our board of directors since February 2015. Mr. Wurzer is currently the Executive Vice President and Chief Investment Officer at Connecticut Innovations, a state-funded venture capital fund, where he previously served as Senior Managing Director and Managing Director. Prior to joining Connecticut Innovations in November 2009, Mr. Wurzer served as Executive Vice President, Treasurer and Chief Financial Officer at CuraGen Corporation from 1997 to 2008. He also held numerous positions at Value Health Inc. from 1991 to 1997, including Senior Vice President, Treasurer and Chief Financial Officer. Mr. Wurzer is a Non-Executive Director of Special Diversified Opportunities, Inc., Thetis Pharmaceutical LLC, My Gene Counsel LLC, Natural Polymer Devices Inc. and Axerion Therapeutics, Inc.; from 2010 to 2012 he was a Non-Executive Director of DUSA Pharmaceuticals, Inc. and from 2010 to 2015 he was a Non-Executive Director of Response Genetics Inc. Mr. Wurzer is a Certified Public Accountant and began his career with Coopers & Lybrand, which is now part of PricewaterhouseCoopers. He received a B.B.A. from the University of Notre Dame. We believe Mr. Wurzer is qualified to serve on our board of directors because of his extensive experience in the pharmaceutical and biotechnology industries and his finance and accounting background.

B. Compensation

The following discussion provides the amount of compensation paid, and benefits in kind granted, by us and our subsidiaries to our current directors and executive officers for services provided in all capacities to us or our subsidiaries for the year ended January 31, 2017, as well as the amount contributed by us into money purchase plans for the year ended January 31, 2017 to provide pension benefits to our current directors and executive officers.

Directors' and Executive Management Compensation

For the year ended January 31, 2017, the table below sets out the compensation paid to our current directors and executive officers.

Compensation for Year Ended January 31, 2017 for Current Directors and Executive Management

Name	Salary and Bonus / Fees	Taxable Benefits(1)	Pension Benefit	Total
Glyn Edwards *Chief Executive Officer and Executive Director*	£609,000	£ 2,226	£17,400	£628,626
Erik Ostrowski *Chief Financial Officer*	$793,800	—	$14,100	$807,900
Frank Armstrong *Non-Executive Chairman*	£ 59,167	£ 904	—	£ 60,071
Barry Price *Non-Executive Director*	£ 30,834	£ 446	—	£ 31,280
Stephen Davies *Non-Executive Director*	£ 31,667	—	—	£ 31,667
Leopoldo Zambeletti *Non-Executive Director*	£ 27,500	£ 302	—	£ 27,802
Valerie Andrews *Non-Executive Director*	£ 50,372	£ 2,584	—	£ 52,956
David Wurzer *Non-Executive Director*	£ 42,954	£ 2,146	—	£ 45,100

(1) Taxable benefits represent the value of the personal benefits granted, which include private medical insurance and life assurance for executive officers and travel costs (and associated income tax and national insurance contributions which were settled on behalf of the Non-Executive Directors) for attendance at board meetings for the Non-Executive Directors. Amounts included are based on the taxable benefits reported in the year ended January 31, 2017 to HM Revenue & Customs.

Total compensation set out in the table above does not include any amounts for the value of options to acquire our ordinary shares granted to or held by the directors and executive management, which is described in "Compensation—Outstanding Equity Awards, Grants and Option Exercise" in this Annual Report.

Bonuses

Our Executive Director and Chief Financial Officer are eligible for annual bonuses at the discretion of our board and, in the case of our Executive Director, our remuneration committee. Annual bonuses are based on achievement of strategic and financial measures and personal performance objectives. Our Executive Director is eligible for annual bonus potential of 150% of his gross base salary to be paid in share options, cash or a combination of both at the discretion of our board. On January 31, 2017, our Executive Director was awarded a bonus representing 110% of his gross base salary, which was paid in cash in February 2017. Mr. Ostrowski, our Chief Financial Officer, is eligible for a discretionary annual bonus in an amount up to 40% of his annual base salary, as determined by our board of directors. On January 31, 2017, Mr. Ostrowski was awarded a bonus representing 110% of his annual base salary. The bonus was paid in cash in February 2017.

Outstanding Options As of January 31, 2017 for Current Directors and Executive Management

Outstanding Equity Awards, Grants and Option Exercise

During the year ended January 31, 2017, options to purchase 360,576 ordinary shares were awarded to our current directors and executive officers. The table below sets out information on outstanding options granted to our current directors and executive officers as of January 31, 2017.

Name	Date of grant	At February 1, 2016	Granted during the period	Lapsed during the period	At January 31, 2017	Price per share (£)	Date from which exercisable	Expiration date
Glyn Edwards	May 10, 2012	150,046	—	—	150,046	0.60	Note 1	May 10, 2022
Chief Executive Officer and Executive Director	May 10, 2012	657,500	—	—	657,500	0.60	Note 2	May 10, 2022
	January 31, 2013	72,973	—	—	72,973	0.20	Note 3	January 31, 2023
	December 18, 2013	300,000	—	(300,000)	—	1.85	Note 4	December 18, 2023
	December 18, 2013	76,364	—	—	76,364	0.20	Note 5	December 18, 2023
	July 15, 2014	600,000	—	—	600,000	1.26	Note 6	July 15, 2024
	June 16, 2015	887,333	—	—	887,333	1.43	Note 11	June 16, 2025
	June 23, 2016	—	110,576	—	110,576	0.01	Note 12	June 23, 2026
		2,744,216	110,576	(300,000)	2,554,792			
Erik Ostrowski	June 23, 2014	400,000	—	—	400,000	1.48	Note 7	June 23, 2024
Chief Financial Officer	June 16, 2015	400,000	—	—	400,000	1.43	Note 11	June 16, 2025
	June 23, 2016	—	250,000	—	250,000	1.05	Note 13	June 23, 2026
		800,000	250,000	—	1,050,000			
Barry Price	April 7, 2011	13,981	—	—	13,981	0.65	Note 8	April 7, 2021
Non-Executive Director	December 18, 2013	25,000	—	(25,000)	—	1.85	Note 4	December 18, 2023
	July 15, 2014	17,500	—	—	17,500	1.26	Note 6	July 15, 2024
	June 16, 2015	25,000	—	—	25,000	1.43	Note 11	June 16, 2025
		81,481	—	(25,000)	56,481			
Frank Armstrong	December 18, 2013	75,000	—	(75,000)	—	1.85	Note 4	December 18, 2023
Non-Executive Director	July 15, 2014	37,500	—	—	37,500	1.26	Note 6	July 15, 2024
	June 16, 2015	50,000	—	—	50,000	1.43	Note 11	June 16, 2025
		162,500	—	(75,000)	87,500			
Stephen Davies	December 18, 2013	25,000	—	(25,000)	—	1.85	Note 4	December 18, 2023
Non-Executive Director	July 15, 2014	17,500	—	—	17,500	1.26	Note 6	July 15, 2024
	June 16, 2015	25,000	—	—	25,000	1.43	Note 11	June 16, 2025
		67,500	—	(25,000)	42,500			
Leopoldo Zambeletti	June 23, 2014	25,000	—	—	25,000	1.48	Note 9	June 23, 2024
Non-Executive Director	June 16, 2015	25,000	—	—	25,000	1.43	Note 11	June 16, 2025
		50,000	—	—	50,000			
Valerie Andrews	December 23, 2014	25,000	—	—	25,000	1.37	Note 10	December 23, 2024
Non-Executive Director	June 16, 2015	25,000	—	—	25,000	1.43	Note 11	June 16, 2025
		50,000	—	—	50,000			
David Wurzer	June 16, 2015	25,000	—	—	25,000	1.43	Note 11	June 16, 2025
Non-Executive Director								
		25,000	—	—	25,000			

1 These options were eligible for full vesting and exercise on and after May 10, 2015, subject to the meeting of performance conditions relating to our ordinary share price. In order to vest in full, the average closing share price of our ordinary shares on AIM needed to be equal to or greater than £2.20 for the two months preceding the third anniversary of the date of the grant, 25% would vest where the average closing share

price is £1.40 and pro-rated where the average closing share price is between £1.41 and £2.19. The options will lapse if the performance condition relating to our average closing share price is not met by the third anniversary of the date of grant. The performance period has now passed and, accordingly, only 150,046 options have vested and 77,454 options have lapsed.

2 The options are split into four tranches with varying performance conditions attached and will only vest if the average closing share price of our ordinary shares on AIM is equal or greater than the specified condition in any period of 60 consecutive calendar days, ending on or before the fifth anniversary of the date of grant. Details of the tranches are as follows: 207,500 with a performance condition based on an average closing share price of £4.00; 200,000 with a performance condition based on an average closing share price of £6.00; 150,000 with a performance condition based on an average closing share price of £8.00; and 100,000 with a performance condition based on an average closing share price of £10.00. The options will lapse if the performance condition is not met by the fifth anniversary of the date of grant.

3 These options were awarded under our bonus incentive. They vested and became exercisable on July 31, 2013.

4 These options failed to meet their performance condition and have lapsed.

5 These options vested and became exercisable on June 18, 2014. These options were awarded as a bonus for the fiscal year ended January 31, 2014 representing 70% of Mr. Edwards' gross basic salary for such fiscal year.

6 These options vested and can be exercised in full on or after the third anniversary of the date of grant.

7 These options vest and become exercisable in the following proportions, assuming the average closing share price of our ordinary shares on AIM during the two months prior to each relevant vesting date is £2.213 or higher: 25% on the second anniversary of the date of grant, 75% on the third anniversary of the date of grant and 100% on the fourth anniversary of the date of grant. These options will lapse if the performance condition is not met by the fourth anniversary of the date of grant.

8 These options were capable of vesting and exercise on or after April 8, 2014 subject to the meeting of performance conditions relating to our share price. In order to vest in full, the average closing share price of our ordinary shares on AIM would have had to exceed £3.00 over the two months ending April 7, 2014. If the performance conditions were not satisfied in full, or in part, the options would lapse in respect of those option shares that did not vest. The performance period has now passed and, accordingly, only 13,981 options have vested and 11,019 options have lapsed since January 31, 2014.

9 These options vest in full subject to (i) completion of Phase 2 proof of concept trials in both the Duchenne muscular dystrophy and Clostridium difficile infection programs or the third anniversary of the date of grant, whichever is sooner and (ii) the average closing share price of our ordinary shares on AIM being equal or greater than £2.213 in any period of 30 consecutive days ending on or before the third anniversary of the date of grant.

10 These options vest if the average closing share price of our ordinary shares on AIM is equal or greater than £2.055 in any period of 30 consecutive days during the period from the date of the grant to September 18, 2017. Once vested, 25% of the options can be exercised on or after September 18, 2016 and all of the options, if vested, can be exercised on or after September 18, 2017. These options will lapse if the performance condition is not met by September 18, 2017.

11 These options vest if the average closing share price of our ordinary shares on AIM is equal or greater than £2.145 in any period of 30 consecutive days during the period from the date of the grant to June 16, 2018. Once vested, a third of the options can be exercised on or after June 16, 2017 and all of the options, if vested, can be exercised on or after June 16, 2018. These options will lapse if the performance condition is not met by June 16, 2018.

12 These options vested and became exercisable on July 21, 2016. These options were awarded as part settlement of the bonus awarded for the fiscal year ended January 31, 2016 representing 50% of Mr. Edwards' gross basic salary for that fiscal year.

13 These options will vest in full on June 23, 2019 subject to achievement of performance conditions pertaining to certain corporate and program development milestones. These options will lapse in full if the performance conditions are not met by June 23, 2019.

We periodically grant share options to employees, including executive officers, to incentivize employees, and align their interests with shareholders. We intend to grant additional options subject to a cap, as previously agreed with shareholders, of up to 15% of total issued share capital in any ten-year period.

Pension Benefits

We operate a defined contribution pension scheme which is available to all employees of our group. For the year ended January 31, 2017, we paid a total of £17,400 in lieu of pension contributions in respect of our Executive Director. In addition, for the year ended January 31, 2017, we made payments of $14,100 into our Chief Financial Officer's 401(k) plan.

Employment Agreements and Letters of Appointment

Non-Executive Directors

Our non-executive directors have each entered into a letter of appointment with us. Each non-executive director's letter of appointment provides for a continuous term for each non-executive director until termination of the letter of appointment. The letters of appointment automatically terminate if the relevant non-executive director is not re-elected to office by the shareholders, is removed from office by a resolution of the shareholders, vacates his or her office, is adjudged bankrupt or enters into any composition or arrangement with his or her creditors, is guilty of misconduct or commits a serious persistent breach of his or her appointment letter, or is unable to perform his or her duties under the appointment for 90 days in aggregate in any period of 12 months. The letters of appointment may also be terminated by mutual agreement or effective immediately upon written notice by one party to the other at any time. Each letter of appointment also includes confidentiality provisions for the protection our confidential information.

Until November 30, 2016, each non-executive director, with the exception of Dr. Armstrong, received £25,000 per annum for payment for services provided to us. Dr. Armstrong received £50,000 per annum, which included payment for services as chairman of our board of directors. Dr. Price and Professor Davies each received an additional £5,000 per annum for each committee they sit on, Mr. Wurzer received an additional £10,000 per annum as payment for services as chair of our audit committee and Ms. Andrews received an additional £5,000 for the committee she sits on and an additional £10,000 per annum as payment for services as chair of our remuneration committee. On and after December 1, 2016, each non-executive director, with the exception of Dr. Armstrong, Mr. Wurzer and Ms. Andrews, received £35,000 per annum for payment for services provided to us. Dr. Armstrong receives £75,000 per annum, which included payment for services as chairman of our board of directors. Mr. Wurzer receives $67,000 per annum and Ms. Andrews receives $77,000 per annum, in each case, as payment for services provided to us and includes payment for services as chair of the audit and remuneration committees. resprectively. Dr. Price and Professor Davies each receive an additional £5,000 per annum for each committee they sit on. Under the letters of appointment, each non-executive director is also entitled to reimbursement for all reasonable expenses incurred in connection with his or her duties as a non-executive director and that are in line with our expense policy.

Executive Director

Glyn Edwards, Chief Executive Officer

Mr. Edwards was appointed as the chief executive officer by a service agreement dated April 4, 2012 which continues unless terminated by us with six months' written notice or by Mr. Edwards with six months' written notice. We may also terminate the agreement with immediate effect by paying a sum in lieu of notice equal to the basic fixed salary which Mr. Edwards would have been entitled to receive during the notice period (and which shall not include payment in respect of benefits). We may otherwise terminate the agreement with immediate effect at any time without notice or payment in lieu of notice for certain circumstances including material breach of the agreement, serious misconduct, serious incompetence or negligence, criminal convictions or bankruptcy.

The agreement includes a garden leave clause for a maximum of two months and there is no provision for compensation in addition to the contractual notice period.

Under his service agreement, Mr. Edwards initially received a salary of £200,000 per annum payable in arrears by equal monthly installments plus reasonable expenses. Effective in February 2017, Mr. Edwards' salary increased to £304,500 per annum. Mr. Edwards' service agreement also provides for a monthly pension contribution equal to 6% of salary, private medical cover (including cover for his spouse) and life assurance (for four times his gross salary). A share option package, as agreed by the chairman of our remuneration committee, will be awarded to Mr. Edwards subject to the rules of our share option scheme. Under his service agreement Mr. Edwards is prohibited from engaging in any type of business in competition with the business of our group, procuring orders from or doing business with any person who has done or proposed to do business with our group, and endeavouring to entice away from our group any senior manager or director engaged by our group, for a period of 12 months from the date of termination of his agreement. Mr. Edwards is also subject to confidentiality and protection of intellectual property provisions.

Executive Management

Erik Ostrowski, Chief Financial Officer

Mr. Ostrowski was appointed as the chief financial officer pursuant to a letter of employment with Summit Therapeutics Inc. dated May 29, 2014 which continues unless terminated by either party at any time with or without notice. Under his letter of employment, Mr. Ostrowski initially received a salary of $330,000 per annum and also received a signing bonus of $50,000. Effective in February 2017, Mr. Ostrowski's salary increased to $400,000 per annum.

Mr. Ostrowski is eligible to receive a discretionary bonus in an amount up to 40% of his annual base salary, as determined by our board of directors. Under his letter of employment, Mr. Ostrowski is reimbursed for medical, dental, vision, life and disability insurance coverage up to an aggregate monthly sum of $1,667 until such time as a group insurance policy is established and is paid a monthly bonus amount of $1,650 until such time as a retirement savings plan for the employees of Summit Therapeutics Inc. is established. As Summit Therapeutics Inc. has implemented a group insurance policy and a retirement savings plan for its employees, Mr. Ostrowski is no longer entitled to these reimbursements. In the event that Mr. Ostrowski's employment is terminated without good cause, he shall receive a severance payment equal to six months of his then-annual base salary plus the value of six months of benefits. Good cause includes willful misconduct, willful or gross neglect of job duties and unauthorized use or disclosure of the group's confidential information.

Mr. Ostrowski has also entered into a confidentiality, inventions, non-compete and non-solicitation agreement dated June 16, 2014 in favor of our group for the protection of our confidential information and intellectual property. Pursuant to that agreement Mr. Ostrowski has also agreed to non-compete and non-solicitation obligations for a period of 12 months following termination of his employment.

David Roblin, Chief Operating Officer and President of Research and Development

Dr. Roblin was appointed as the chief operating officer and president of research and development pursuant to a letter of employment with Summit (Oxford) Limited dated November 25, 2016 which became effective on December 16, 2017 and continues unless terminated by either party at any time with or without notice. Under his letter of employment, Dr. Roblin initially will receive a salary of £300,000 per annum, and he is also entitled to an additional £30,000 per annum for the purposes of assuring a second place of residence for Dr. Roblin near Oxford.

Dr. Roblin's employment agreement provides for a monthly pension contribution (or payments in lieu thereof) equal to 6.0% of salary, private medical coverage for him and his spouse and life insurance coverage equal to an amount that is four times his gross base salary. A share option package equal to twice his base salary, as agreed

by the chairman of our remuneration committee, will be awarded to Dr. Roblin at the first available opportunity after he begins working for us full-time, subject to the rules of our share option scheme. Under his employment agreement Dr. Roblin is prohibited from engaging in any type of business in competition with the business of our group, procuring orders from or doing business with any person who has done or proposed to do business with our group, and endeavouring to entice away from our group any senior manager or director engaged by our group, for a period of 6 months from the date of termination of his letter of employment. Dr. Roblin is also subject to confidentiality and protection of intellectual property obligations under his employment letter.

Dr. Roblin is eligible to receive a discretionary bonus in an amount up to 50% of his annual base salary and an annual share option award subject to performance conditions, in each case, as determined by our board of directors.

Equity Compensation Arrangements

2016 Long Term Incentive Plan

Our 2016 Long Term Incentive Plan, which we refer to as the Incentive Plan, was adopted on January 21, 2016. Under the Incentive Plan our board may grant conditional awards, options, cash conditional awards and cash options to any of our employees, including executive directors, and the employees of our subsidiaries. The Incentive Plan is administered by our board, which has full authority, consistent with the Incentive Plan, to administer the Incentive Plan, including the authority to interpret and construe any provision of the Incentive Plan and to adopt regulations for administering the Incentive Plan. Decisions of the board are final and binding on all parties. References to our board in this summary shall include any duly authorized committee of our board.

Our board may grant awards to any employees eligible to receive awards under the Incentive Plan in its discretion, subject to the rules of the Incentive Plan and such additional terms as the board may determine. However, the grant of an award is subject to obtaining any approval or consent required by any relevant authority and any other applicable laws or regulations. Awards must be granted by deed (or in such other written form as the board determines) and, as soon as reasonably practicable after the date on which an award is granted, the participant must be notified of the terms of his or her award.

A conditional award is a right to acquire shares subject to and in accordance with the rules of the Incentive Plan with no exercise period. An option is a right to acquire shares subject to and in accordance with the rules of the Incentive Plan during a specified exercise period not to exceed ten years from the date the option is granted. A cash conditional award is a right to receive a cash payment equal to the market value (as determined by the board) of a number of notional shares underlying the vested portion of the award on the vest date. A cash option is a right to receive a cash payment equal to the market value (as determined by the board) of a number of notional shares underlying the vested portion of the award the date of exercise less the aggregate exercise price payable (if any).

Vesting of Awards

Unless the board determines otherwise, the vesting of an award granted under the Incentive Plan is subject to the satisfaction of a performance condition. Subject to the terms of the Incentive Plan that apply upon a cessation of a participant's employment and upon certain corporate events, the performance condition will be measured over the performance period which, unless the board determines otherwise, will be at least three years. Performance conditions may be amended or substituted by the board if one or more events occur which cause the board to consider that an amended or substituted performance condition would be more appropriate and would not be materially less difficult to satisfy than the original performance condition to which the award was subject.

As soon as reasonably practicable after the end of the performance period relating to an award that is subject to a performance condition, our board will determine if and to what extent the performance condition has been satisfied. To the extent that the performance condition has not been satisfied in full, the remainder of the award will lapse immediately. Subject to the terms of the Incentive Plan that apply upon a cessation of a participant's

employment and upon certain corporate events, an award that is subject to a performance condition will vest on the later of the date on which the board determines that the performance condition has been satisfied and the third anniversary of the date of grant of the award (or such other date determined by the board and communicated to the participant). An award that is not subject to the satisfaction of a performance condition will vest on the third anniversary of the date of grant (or such other date determined by the board and communicated to the participant). We refer to the date on which an award would normally vest, whether or not it is subject to the achievement of a performance condition, as the normal vesting date. Notwithstanding the foregoing, if there are share dealing restrictions (imposed by the company's share dealing code, the rules of the London Stock Exchange, or any other applicable laws or regulations) on the applicable normal vesting date, the award will vest on the date the dealing restrictions are lifted.

The Incentive Plan provides that if a participant dies prior to the date on which an award vests, a number of shares subject to such award will, unless the board determines otherwise, vest as soon as practicable following the participant's death. The number of shares that vest in such circumstance will depend, unless the board determines otherwise, on the extent to which any applicable performance condition has been satisfied at the date of death and the period of time that has elapsed since the start of the applicable performance period or, if the award is not subject to a performance condition, since the date of grant of the award, or such other period as the board determines. To the extent that an award does not vest in full, the remainder will lapse immediately.

In addition, the Incentive Plan provides that if a participant ceases to hold office or employment with any group member (as such term is defined in the Incentive Plan) prior to the date on which an award vests as a result of ill-health, injury or disability; redundancy or retirement; upon the company for which the participant works ceasing to be a group member or the transfer of an undertaking or part-undertaking in which the participant is employed to a company not in our group; or any other reason at the board's discretion (except where a participant is summarily dismissed), the board may determine that an award will vest as soon as practicable following the date of the participant's cessation of office or employment (or on such other date as determined by the board). Otherwise, the award will vest on the normal vesting date. In either case, the number of shares that will vest will depend, unless the board determines otherwise, on the extent to which any applicable performance condition has been satisfied at the date of cessation of office or employment and the period of time that has elapsed since the start of the applicable performance period or, if the award is not subject to a performance condition, since the date of grant of the award, or such other period as the board determines. To the extent that an award does not vest in full, the remainder will lapse immediately. If a participant ceases to hold office or employment with a group member for any other reason prior to the vesting date, his or her award will lapse at such time.

Exercise of Options

Generally, options must be exercised while the participant holds office or employment with a member of our group. In the event, however, that a participant ceases to hold office or employment with a member of our group as a result of ill-health, injury or disability; redundancy or retirement; upon the company for which the participant works ceasing to be a member of our group or the transfer of an undertaking or part-undertaking in which the participant is employed to a company not in our group; or any other reason at the board's discretion (except where a participant is summarily dismissed) prior to the date on which the award becomes exercisable, the option may be exercised, subject to it lapsing upon certain corporate events, for a period of six months (or such other period as the board may determine) commencing on the date the award vests (as described above). If a participant ceases to hold office or employment with a member of our group on or after the vesting date of the option as a result of the participant's resignation or an event described in the preceding sentence on or after the date on which the award becomes exercisable, the option may be exercised, subject to it lapsing upon certain corporate events, for a period of six months (or such other period as the board may determine) from the date of such cessation.

If a participant dies before his or her vested option has been exercised, the participant's personal representatives may exercise the option for 12 months (or such other period as the board may determine) after the later of the date of the participant's death and the date on which the award becomes exercisable.

All awards lapse in prescribed circumstances, including upon the tenth anniversary of the date of grant; the expiry of the period (if any) allowed for the satisfaction of any performance condition without such condition having been satisfied; on the day on which a participant ceases to hold office or employment with a group member (with the exception of the carve outs detailed in the Incentive Plan and described above); the expiry of the period during which an option may be exercised following the participant's death or cessation of office or employment with a group member; on the bankruptcy of the participant; or at such time the participant attempts to transfer, assign, charge or otherwise dispose of his or her award in any way (other than in the event of the participant's death, to his personal representatives).

Dividend Equivalent

The board may decide at any time prior to the issue or transfer of the shares in respect of an award that has vested that the participant will receive an amount (in cash and/or additional shares) equal in value to any dividends that would have been paid on those shares on such terms and over such period (ending no later than the vesting date of the award) as the board may determine. This amount may assume the reinvestment of dividends (on such basis as the board may determine) and may exclude or include special dividends.

Cash Equivalent/Net Settlement

Unless our board has determined that this rule will not apply to all or any portion of an award, at any time prior to the date on which shares in respect of an award that has been vested or exercised have been issued or transferred to a participant, the board may determine that the participant will receive (i) in lieu of ordinary shares, a cash payment equal to the market value (as determined by the board) of the number of shares that would otherwise have been issued or transferred to the participant, less, in the case of an option, the aggregate exercise price payable (if any) or (ii) a reduced number of shares, which reduced number of shares will be equal to the market value (as determined by the board) of the number of shares that would otherwise have been issued or transferred to the participant, less (if the board so determines) any deductions (including, but not limited to any tax or similar liabilities) as may be required by law in respect of the award and, in the case of an option, the aggregate exercise price payable (if any).

Limits

The board may not grant an award that would cause the number of ordinary shares allocated under the Incentive Plan and under any other employee share plan adopted by the company (including the 2005 EMI Scheme described below) to exceed a number equal to 15% of our ordinary share capital in issue at that time. The Incentive Plan sets forth rules for determining when shares are treated as allocated under the Incentive Plan and makes clear that the number of shares allocated does not include shares in respect of which the right to acquire such shares lapses or is released or shares allocated in respect of awards which are then satisfied in cash.

The Incentive Plan also includes an individual participant limit: no eligible employee may be granted an award which would, at the time of grant, cause the market value (as determined by the board) of all the shares subject to awards granted to the participant in a particular financial year of the company to exceed ten times the participant's annual base salary. If any award exceeds this limit, it will be scaled back accordingly.

Reduction of Awards and Clawback

The board may, in its discretion, determine at any time prior to the vesting of an award to reduce (including to zero) the number of shares to which an award relates and/or impose further conditions on an award where there has been a material misstatement of our group's audited financial results; a material failure of risk management by us; serious reputational damage to us, any member of our group or a relevant business unit; material misconduct on the part of the participant or any other circumstances which the board considers to be similar in their nature or effect.

The board may also, in its discretion, determine that at any time after the vesting of an award prior to the later of the second anniversary of vesting and the fifth anniversary of the date of grant (or such longer period as is required by SEC rules that are applicable to us) to take the action described in the preceding paragraph (if an option has not yet been exercised or if shares or cash have not yet been delivered to the participant following the vesting of a conditional award or exercise of an option); require a participant or former participant to make a cash payment to us in respect of some or all of the shares or cash delivered to him or her under the award; and/or require a participant or former participant to transfer for no consideration some or all of the shares delivered to him or her under the award, where there has been a material misstatement of our group's audited financial results or material misconduct on the part of the participant. The board will have discretion to determine the basis on which the amount of cash or shares is calculated including whether, and the extent to which, any tax or social security liability is applicable to the award.

The board may decide to reduce (including to zero) the number of shares to which an award relates or may relate; impose further conditions on an award; and/or require a participant to transfer for no consideration some or all of the shares delivered to such participant under an award or make a cash payment to us in respect of some or all of the shares delivered to such participant under an award to effect the recovery of sums paid or shares delivered under any provisions similar to the rules described in the preceding paragraphs which are included in any bonus plan or share plan (other than the Incentive Plan) operated by any member of our group.

Corporate Events

In certain specified circumstances involving a change of control of our company, all awards which have not yet vested will vest at the time of the change of control or an earlier date, to the extent determined by the board in its discretion, taking into account, unless the board determines otherwise, the extent to which any performance condition has, in the board's opinion, been satisfied and the period of time that has elapsed from the grant date to the date of the change of control (or the date of cessation of office or employment, if earlier). To the extent an award does not vest (or is not exchanged, as described below) it will lapse immediately. Vested options will be exercisable for three months (or such other period as our board may determine) from the date of the change of control, after which time all options will lapse. Notwithstanding the foregoing, an award will not vest but will be exchanged for a new award which, in the opinion of the board, is equivalent to the original award, but relates to shares in a different company, if an offer to exchange the award is made and accepted by the participant; there is an Internal Reorganisation (as that term is defined in the Incentive Plan) unless the board determines that the award should nevertheless vest as described above upon the Internal Reorganisation; or the board decides (before the relevant event) that an existing award will automatically be exchanged.

Awards may also vest upon or become exercisable for a specified period following the occurrence of certain other corporate events, including, for example, if a person becomes bound or entitled to acquire shares pursuant to certain provisions of U.K. company law; if the company is affected by a demerger, delisting, special dividend or other event. The board may also provide for the vesting of awards that have not yet vested or permit exercise of vested options within a specified period following the date on which we pass a resolution for a voluntary winding up of our company.

Variation of Share Capital

In the event of any variation of the share capital of the company or a demerger, delisting, special dividend or other event which may, in the opinion of the board, affect the current or future value of shares, the number of shares that may be allocated under the Incentive Plan, the number of shares subject to an award, any performance condition and/or the exercise price of an option may be adjusted in such manner as the board determines.

Amendments

The board may at any time amend the rules of the Incentive Plan or, except as otherwise provided in the Incentive Plan, the terms of any award. An amendment that is to the material disadvantage of the existing rights

of a participant will not be made unless the participant has approved the amendment and no amendment will be made that would prevent the Incentive Plan from being an employees' share scheme under U.K. law.

Termination

The Incentive Plan will terminate, and no award may be granted under the Incentive Plan, after the tenth anniversary of adoption by the Board. The Incentive Plan may also be terminated at any earlier time by the passing of a resolution by the board or an ordinary resolution of the company in general meeting. Termination of the Incentive Plan will be without prejudice to the existing rights of participants.

Schedule 2—Company Share Option Plan

The purpose of the Company Share Option Plan, or CSOP, is to enable us to grant CSOP options to eligible U.K. employees in accordance with the Income Tax (Earnings and Pensions) Act 2003, Schedule 4 (commonly known as the Enterprise Management Incentive Scheme provisions). Substantially all the rules of the Incentive Plan apply to CSOP options but there are a number of differences, including that there is no cash or dividend equivalent or ability to net settle a CSOP option; the eligibility rules are more limited; and the value of awards under the CSOP is subject to lower limits than those in the Incentive Plan.

Schedule 3—U.S. Participants

We have in place rules governing awards granted to our U.S. employees which have been adapted from the Incentive Plan. The rules are substantially the same as the Incentive Plan, but are designed to ensure that awards granted under the Incentive Plan comply with or are exempt from Section 409A of the U.S. Internal Revenue Code.

2005 EMI Scheme Rules

Our 2005 EMI Scheme Rules were adopted on December 1, 2005. Under the scheme we may grant enterprise management incentive options, known as approved options, to those eligible bona fide employees and directors who qualify under applicable U.K. tax law and, to the extent that our employees and directors do not qualify for approved options, unapproved options may be granted to such eligible bona fide employees and directors. Options can no longer be granted under this scheme.

Exercise of Options

Vesting of options is subject to such performance conditions as shall be set out in the agreement granting an option pursuant to the scheme and shall be otherwise determined by the board in accordance with the scheme. An approved option must be capable of being exercised within the period of ten years from the date of grant. Performance conditions may be amended, relaxed or waived by us if an event occurs which would cause us to consider that an amended performance condition would be a fairer measure of performance provided that such amended targets are no more and no less difficult to satisfy than they were prior to amendment.

Generally, options must be exercised while the participant is an eligible employee or director. In the event, however, that a participant ceases to be an eligible employee or director as a result of ill-health, injury, or disability; redundancy, retirement or pregnancy; upon the company for which the participant works ceasing to be a member of our group; or the transfer of an undertaking or part-undertaking in which the participant is employed to a company not in our group, the option may be exercised during the period commencing on the date he ceases to be an eligible employee or director and ending on 12 months thereafter. If a participant dies while he is an eligible employee or director, the participant's personal representatives may exercise the option for 12 months after the participant's death. All options lapse in prescribed circumstances, including: upon the tenth anniversary of the date of grant; the expiry of the period (if any) allowed for the satisfaction of any performance condition

without such condition having been satisfied or becomes, in our opinion, incapable of being satisfied; on the day on which a participant ceases to be an eligible employee or director (with the exception of the carve outs detailed in the scheme); on the bankruptcy of the participant; or on the occurrence of a takeover.

Ordinary shares allotted under the scheme rank equally with the ordinary shares in issue at the date of allotment of the option shares. If and for so long as the ordinary shares are listed on AIM or any other exchange, we shall apply for ordinary shares allotted under the scheme to be admitted to the relevant exchange.

Limits

The maximum number of ordinary shares which may on any day be placed under option under the scheme, when added to the number of ordinary shares allocated for subscription for the preceding ten years under any employee share scheme, shall not exceed 15% of our ordinary share capital immediately prior to that day. Approved options are also subject to individual participant limits in accordance with the scheme and as provided for under relevant U.K. tax law. Lapsed options shall be disregarded for these purposes.

Takeovers and Liquidations

In certain specified circumstances involving a change of control, as specified in accordance with U.K. tax law, an option may automatically vest or otherwise be determined to vest by our board of directors. Where an option vests by reason of a change of control, the exercise of the option shall be conditional upon the change of control occurring. Our board of directors may, in certain circumstances, determine that an option shall lapse upon the change of control or six months thereafter.

Options may also be exercisable for the relevant period in the event of certain court sanctioned restructurings or amalgamations of us or if another company becomes bound or entitled to acquire our ordinary shares pursuant to certain provisions of U.K. companies law. Our board of directors may also permit exercise of the options within a period following the date on which we pass a resolution for voluntary winding up.

In the event of a person obtaining control of us as a result of a takeover offer or court sanctioned restructuring or amalgamation or qualifying exchange of shares within the relevant U.K. laws, the participant may, by agreement with the acquiring company, release options in consideration for the grant of a new option with respect to the acquiring company's shares.

Variation of Share Capital

In the event of any capitalization, rights issue, consolidation, subdivision, reduction or other variation of our share capital the number of ordinary shares comprised in an option and the exercise price in respect of the ordinary shares shall be varied as the directors determine and our auditors confirm to be fair and reasonable. Limitations apply to the extent to which any such adjustments may reduce the price at which ordinary shares may be purchased pursuant to the exercise of an option and no adjustment will take effect until it has been approved by the United Kingdom tax authorities in accordance with applicable U.K. tax law.

Amendments

Our board of directors may waive or amend the scheme subject to certain limitations which require approval of our shareholders.

Scheme Rules Governing Options Awarded to U.S. Employees

We have in place rules governing options awarded to our U.S. employees which have been adapted from our 2005 EMI Scheme Rules. The rules of the scheme are substantially the same as the 2005 EMI Scheme Rules.

Options Granted Outside the 2005 EMI Scheme Rules

Certain of our consultants who are not eligible employees of companies in our group for the purposes of our option scheme rules, and therefore, are not eligible to participate in our option schemes as detailed above, have been granted options to acquire our shares pursuant to separate unapproved option agreements. These options are generally on comparable terms to options granted under the 2005 EMI Scheme Rules.

Limitations on Liability and Indemnification Matters

To the extent permitted by the U.K. Companies Act 2006, we are empowered to indemnify our directors against any liability they incur by reason of their directorship. We maintain directors' and officers' insurance to insure such persons against certain liabilities. We have entered into a deed of indemnity with each of our directors and executive officers.

C. Board Practices

Board Composition

Our board of directors currently consists of seven members, a non-executive chairman, one executive director and five non-executive directors.

Under NASDAQ listing standards, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. Our board of directors has determined that all of our directors, other than Mr. Edwards, qualify as independent directors under Rule 5605(a)(2) of the NASDAQ listing standards.

Corporate Governance and Committees of the Board

Corporate Governance

Our board of directors is responsible for overall corporate governance and for supervising the general affairs and business of our company and its subsidiaries. As an AIM-listed company, we are subject to the continuing requirements of the AIM Rules for Companies as published by the London Stock Exchange plc from time to time. We are not required to comply with the U.K. Corporate Governance Code by virtue of being an AIM-listed company. Our board, however, seeks to apply the highest standards of corporate governance appropriate for the size and stage of development of the company.

Our board is responsible to our shareholders for the proper management of our company and its subsidiaries and setting the overall direction and strategy of our group, reviewing scientific, operational and financial performance, and advising on management appointments. All key operational and investment decisions are subject to board approval.

There is a clear separation of the roles of chief executive officer and non-executive chairman. The chairman is responsible for overseeing the running of our board, ensuring that no individual or group dominates our board's decision-making and ensuring that the non-executive directors are properly briefed on matters. The chief executive officer has the responsibility for implementing the strategy of our board and managing the day-to-day business activities of our group.

Under our articles of association, all of our directors are subject to election by shareholders at the first annual general meeting after their appointment to our board and to re-election by shareholders at least once every three years. Accordingly we plan to put one-third of the directors up for re-election each year and plan to put two of the directors up for re-election at the 2017 annual general meeting. The board considers a classified board structure and the practice of retiring by rotation every three years to be appropriate given that, as a

biopharmaceutical company, the nature of our business is to carry out long-term research and development. Further details regarding the directors to be proposed for re-election will be detailed in the 2017 notice of annual general meeting that will be distributed to shareholders in accordance with our articles of association.

Committees of the Board

We have established an audit committee, a remuneration committee and a nominating and corporate governance committee and have adopted a charter for each of these committees.

Audit Committee

The members of our audit committee are Mr. Wurzer, Dr. Price and Ms. Andrews. Mr. Wurzer is the chair of the audit committee. Our audit committee's responsibilities include:

- appointing, approving the compensation of, and assessing the independence, objectivity and effectiveness of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from that firm;
- monitoring the integrity of our financial statements by reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- reviewing and monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct;
- reviewing and monitoring the effectiveness of our internal audit function;
- overseeing our risk assessment and risk management policies;
- establishing policies regarding procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our internal auditing staff, if any, our independent registered public accounting firm and management; and
- reviewing and approving or ratifying any related person transactions.

All audit and non-audit services, other than *de minimis* non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

Our board of directors has determined that Mr. Wurzer is an "audit committee financial expert" as defined in Item 16A of Form 20-F.

In order to satisfy the independence criteria for audit committee members set forth in Rule 10A-3(b)(1) under the Exchange Act, each member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. We believe that the composition of our audit committee meets the requirements for independence under current NASDAQ and SEC rules and regulations.

Remuneration Committee

The members of our remuneration committee are Dr. Armstrong, Ms. Andrews and Professor Davies. Ms. Andrews is the chair of the remuneration committee. Our remuneration committee's responsibilities include:

- reviewing and approving, or making recommendations to our board of directors with respect to, the compensation of our directors and executive management;
- overseeing an evaluation of our executive management; and
- overseeing and administering our employee share option scheme or equity incentive plans in operation from time to time.

In order to satisfy the independence criteria for remuneration committee members set forth in Rule 10C-1 under the Exchange Act, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a remuneration committee member must be considered, including, but not limited to: (1) the source of compensation of the director, including any consulting advisory or other compensatory fee paid by such company to the director; and (2) whether the director is affiliated with the company or any of its subsidiaries or affiliates. We believe the composition of our remuneration committee meets the requirements for independence under current NASDAQ and SEC rules and regulations.

Nominating and Corporate Governance Committee

The members of our nominating and corporate governance committee are Dr. Armstrong, Dr. Price, Professor Davies, Mr. Zambeletti, Ms. Andrews and Mr. Wurzer. Dr. Armstrong is the chair of the nominating and corporate governance committee. Our nominating and corporate governance committee's responsibilities include:

- identifying individuals qualified to become members of our board;
- recommending to our board the persons to be nominated for election as directors and to each of our board's committees;
- overseeing a periodic evaluation of our board;
- reviewing and making recommendations to our board with respect to our board leadership structure;
- reviewing and making recommendations to our board with respect to management succession planning; and
- developing and recommending to our board corporate governance principles.

D. Employees

The number of our employees by geographic location and function as of the end of the period for our fiscal years ended January 31, 2017, 2016 and 2015 was as follows:

	2017	2016	2015
By Geography			
United Kingdom	28	25	21
North America	12	12	4
Total	40	37	25

	2017	2016	2015
By Function			
Research & Development	24	22	15
General & Administrative	16	15	10
Total	40	37	25

Our employees are not represented by any collective bargaining agreements, and we have never experienced a work stoppage. We believe our employee relations are good.

Item 7: Major Shareholders and Related Party Transactions

A. Major Shareholders

The following table sets forth information with respect to the beneficial ownership of our ordinary shares as of March 15, 2017 by:

- each of the members of our board of directors;
- each of our other executive officers; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our ordinary shares.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our ordinary shares. Our ordinary shares subject to options or warrants that are currently exercisable or exercisable within 60 days of March 15, 2017 are considered outstanding and beneficially owned by the person holding the options or warrants for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise noted, the persons and entities in this table have sole voting and investing power with respect to all of the ordinary shares beneficially owned by them. Except as otherwise indicated in the table below, addresses of named beneficial owners are c/o Summit Therapeutics plc, 136a Eastern Avenue, Milton Park, Abingdon, Oxfordshire OX14 4SB, United Kingdom. All holders of our ordinary shares, including those shareholders listed below, have the same voting rights with respect to such shares.

	Ordinary shares beneficially owned	
Name of beneficial owner	Shares	%
Executive officers and directors		
Glyn Edwards(1)	843,292	1.35%
Erik Ostrowski	—	—
Frank Armstrong(2)	26,942	*
Barry Price(3)	95,544	*
Stephen Davies(4)	590,814	*
Leopoldo Zambeletti	—	—
Valerie Andrews	10,500	*
David Wurzer	7,500	*
All executive officers and directors as a group (8 persons)(5)	1,574,592	2.54%
5% shareholders		
Lansdowne Partners (UK) LLP(6)	15,727,170	25.41%
Point72 Asset Management, L.P.(7)	4,630,995	7.48%
Robert Keith(8)	4,294,816	6.94%

* Less than one percent.

(1) Consists of (a) 609,959 ordinary shares underlying options that are exercisable as of March 15, 2017 or will become exercisable within 60 days after such date and (b) 233,333 ordinary shares.
(2) Consists of (a) 12,500 ordinary shares underlying options that are exercisable as of March 15, 2017 or will become exercisable within 60 days after such date and (b) 14,442 ordinary shares.
(3) Consists of (a) 19,814 ordinary shares underlying options that are exercisable as of March 15, 2017 or will become exercisable within 60 days after such date and (b) 75,730 ordinary shares.
(4) Consists of (a) 5,833 ordinary shares underlying options that are exercisable as of March 15, 2017 or will become exercisable within 60 days after such date and (b) 584,981 ordinary shares.
(5) Consists of (a) 648,106 ordinary shares underlying options that are exercisable as of March 15, 2017 or will become exercisable within 60 days after such date and (b) 926,486 ordinary shares.
(6) These shares are registered in the name of HSBC Client Holdings Nominee (UK) Limited. Lansdowne Partners (UK) LLP may be deemed to have voting and dispositive power over the ordinary shares. Investment decisions with respect to the ordinary shares held by Lansdowne Partners (UK) LLP can be made by Stuart Roden, Peter Davies and Jonathan Regis. The address of Lansdowne Partners (UK) LLP is 15 Davies Street, London, W1K 3AG.
(7) This information is based on a Schedule 13G filed on February 10, 2017 by Point72 Asset Management, L.P., Point72 Capital Advisors, Inc. and Steven A. Cohen. Each of Point72Asset Management, L.P., Point72 Capital Advisors, Inc. and Mr. Cohen reported that it or he has shared voting and dispositive power with respect to 4,630,995 of our ordinary shares held by certain investment funds managed by Point72Asset Management, L.P. Point72 Capital Advisors, Inc. is the general partner of Point72 Asset Management, L.P., and Mr. Cohen controls Point72 Capital Advisors, Inc. The address provided therein for Point72 Asset Management, L.P., Point72 Capital Advisors, Inc. and Mr. Cohen is 72 Cummings Point Road, Stamford, CT 06902. Each of Point72 Asset Management, L.P., Point72 Capital Advisors Inc. and Mr. Cohen disclaims beneficial ownership of such shares.
(8) This information is based on information contained in a TR-1 Notification send to us on January 25, 2017 by Robert Keith.

Bank of New York Mellon, or BNY Mellon, is the holder of record for the company's ADR program, pursuant to which each ADS represents five ordinary shares. As of March 15, 2017, BNY Mellon held 23,569,880 ordinary shares representing 38.1% of the issued share capital held at that date. As of March 15, 2017, we had one holder of record with an addresse in the United States, and such holder held less than one percent of our outstanding ordinary shares. As a result, the number of holders of record or registered holders in the United States is not representative of the number of beneficial holders or of the residence of beneficial holders.

To our knowledge, there has been no significant change in the percentage ownership held by the principal shareholders listed above since March 15, 2017.

B. Related Party Transactions

Since February 1, 2016, we have not engaged in any transactions with our directors, executive officers or holders of 5% or more of our ordinary shares, or affiliates of our directors, executive officers or holders of more than 5% of our ordinary shares that are required to be described in this Annual Report pursuant to Item 7.B. of Form 20-F.

C. Interests of Experts and Counsel

Not applicable.

Item 8: Financial Information

A. Consolidated Financial Statements and Other Financial Information.

See "Item 18. Financial Statements."

B. Significant Changes.

There have been no significant changes since January 31, 2017.

C. Dividends

We have never declared or paid any dividends and currently intend to retain all available earnings generated by our operations for the development and growth of our business. We do not currently anticipate paying any cash dividends on our shares.

Item 9: The Listing

A. Listing Details

Our ordinary shares have been trading on AIM, a market operated by the London Stock Exchange plc, or AIM, under the symbol "SUMM" since October 14, 2004.

The following table sets forth, for the periods indicated, the reported high and low closing sale prices of our ordinary shares on AIM in pounds sterling and U.S. dollars. Price per ordinary share in U.S. dollars amounts below have been translated into U.S. dollars at the noon buying rate of the Federal Reserve Bank of New York on January 31, 2017 of £1.00 to $1.2585.

	Price Per Ordinary Share £		Price Per Ordinary Share $	
	High	Low	High	Low
Annual (Fiscal Year Ended January 31):				
2013	1.65	0.45	2.08	0.57
2014	3.90	0.78	4.91	0.98
2015	2.20	1.04	2.77	1.31
2016	1.84	1.17	2.32	1.47
2017	2.53	0.91	3.18	1.15
Quarterly:				
First Quarter 2016	1.84	1.36	2.32	1.71
Second Quarter 2016	1.61	1.33	2.03	1.67
Third Quarter 2016	1.63	1.29	2.05	1.62
Fourth Quarter 2016	1.50	1.17	1.89	1.47
First Quarter 2017	1.33	0.94	1.67	1.18
Second Quarter 2017	1.26	1.00	1.59	1.26
Third Quarter 2017	2.53	0.91	3.18	1.15
Fourth Quarter 2017	1.95	1.43	2.45	1.80
Monthly:				
September 2016	1.50	0.91	1.89	1.15
October 2016	2.53	1.31	3.18	1.65
November 2016	1.95	1.68	2.45	2.11
December 2016	1.73	1.43	2.18	1.80
January 2017	1.95	1.43	2.45	1.80
February 2017	1.98	1.83	2.49	2.30
March 2017 (through March 27, 2017)	2.20	2.00	2.77	2.52

On March 27, 2017, the last reported sales price of our ordinary shares on AIM was £2.13 per ordinary share ($2.58 per ordinary share).

Our American Depositary Shares, or ADSs, have been trading on the NASDAQ Global Market under the symbol "SMMT" since March 5, 2015. The following table sets forth, for the periods indicated, the reported high and low closing sale prices of our ADSs on the NASDAQ Global Market in U.S. dollars.

	Price Per ADS $	
	High	Low
Annual (Fiscal Year Ended January 31):		
2016	13.68	8.25
2017	14.35	5.50
Quarterly:		
First Quarter 2016 (from March 5, 2015)	13.68	10.19
Second Quarter 2016	13.00	10.13
Third Quarter 2016	13.29	10.10
Fourth Quarter 2016	11.80	8.25
First Quarter 2017	10.97	6.35
Second Quarter 2017	9.84	7.00
Third Quarter 2017	14.35	5.50
Fourth Quarter 2017	12.08	8.12
Monthly:		
September 2016	10.02	5.50
October 2016	14.35	8.65
November 2016	12.08	9.83
December 2016	10.27	8.12
January 2017	11.85	8.47
February 2017	12.23	10.76
March 2017 (through March 27, 2017)	13.21	12.11

On March 27, 2017, the last reported sales price of our ADSs on the NASDAQ Global Market was $12.62 per ADS.

B. Plan of Distribution

Not applicable.

C. Markets

Our ordinary shares are listed on AIM under the symbol "SUMM" and our ADSs are listed on the NASDAQ Global Market under the symbol "SMMT."

D. Selling Shareholders

Not applicable.

E. Dilution

Not applicable.

F. Expenses of the Issue

Not applicable.

Item 10: Additional Information

A. Share Capital

Not applicable.

B. Memorandum and Articles of Association

We incorporate by reference into this Annual Report the description of our amended articles of association contained in our Registration Statement on Form F-1 (File No. 333-201807) originally filed with the SEC on January 30, 2015, as amended.

C. Material Contracts

Except as otherwise disclosed in this Annual Report (including the exhibits hereto), we are not currently, and have not been in the last two years, party to any material contract, other than contracts entered into in the ordinary course of business.

D. Exchange Controls

There are no governmental laws, decrees, regulations or other legislation in the United Kingdom that may affect the import or export of capital, including the availability of cash and cash equivalents for use by us, or that may affect the remittance of dividends, interest, or other payments by us to non-resident holders of our ordinary shares or ADSs, other than withholding tax requirements. There is no limitation imposed by English law or our articles of association on the right of non-residents to hold or vote shares.

E. Taxation

Taxation in the United Kingdom

The following is a general summary of certain U.K. tax considerations relating to the ownership and disposal of our ordinary shares or ADS and does not address all possible tax consequences relating to an investment in our ordinary share or ADS. It is based on U.K. tax law and generally published HM Revenue & Customs, or HMRC, practice as of the date of this Annual Report, both of which are subject to change, possibly with retrospective effect.

Save as provided otherwise, this summary applies only to a person who is the absolute beneficial owner of our ordinary share or ADS and who is resident (and, in the case of an individual, domiciled) in the United Kingdom for tax purposes and who is not resident for tax purposes in any other jurisdiction and does not have a permanent establishment or fixed base in any other jurisdiction with which the holding of our ordinary share or ADS is connected ("U.K. Holders"). A person (a) who is not resident (or, if resident, is not domiciled) in the United Kingdom for tax purposes, including an individual and company who trades in the United Kingdom through a branch, agency or permanent establishment in the United Kingdom to which an ordinary share or ADS is attributable, or (b) who is resident or otherwise subject to tax in a jurisdiction outside the United Kingdom, is recommended to seek the advice of professional advisors in relation to their taxation obligations.

This summary is for general information only and is not intended to be, nor should it be considered to be, legal or tax advice to any particular investor. It does not address all of the tax considerations that may be relevant to specific investors in light of their particular circumstances or to investors subject to special treatment under U.K. tax law. In particular:

- this summary only applies to an absolute beneficial owner of ordinary share or ADS and any dividend paid in respect of the ordinary share where the dividend is regarded for U.K. tax purposes as that person's own income (and not the income of some other person); and
- this summary: (a) only addresses the principal U.K. tax consequences for an investor who holds ordinary share or ADS as a capital asset, (b) does not address the tax consequences that may be

relevant to certain special classes of investor such as a dealer, broker or trader in shares or securities and any other person who holds ordinary share or ADS otherwise than as an investment, (c) does not address the tax consequences for a holder that is a financial institution, insurance company, collective investment scheme, pension scheme, charity or tax-exempt organization, (d) assumes that a holder is not an officer or employee of the company (nor of any related company) and has not (and is not deemed to have) acquired the ordinary share or ADS by virtue of an office or employment, and (e) assumes that a holder does not control or hold (and is not deemed to control or hold), either alone or together with one or more associated or connected persons, directly or indirectly (including through the holding of an ADS), an interest of 10% or more in the issued share capital (or in any class thereof), voting power, rights to profits or capital of the company, and is not otherwise connected with the company.

This summary further assumes that a holder of ADSs is the beneficial owner of the underlying ordinary share for U.K. direct tax purposes.

POTENTIAL INVESTORS IN THE ADSs SHOULD SATISFY THEMSELVES PRIOR TO INVESTING AS TO THE OVERALL TAX CONSEQUENCES, INCLUDING, SPECIFICALLY, THE CONSEQUENCES UNDER U.K. TAX LAW AND HMRC PRACTICE OF THE ACQUISITION, OWNERSHIP AND DISPOSAL OF THE ORDINARY SHARES OR ADSs, IN THEIR OWN PARTICULAR CIRCUMSTANCES BY CONSULTING THEIR OWN TAX ADVISERS.

Taxation of Dividends

Withholding Tax

A dividend payment in respect of an ordinary share may be made without withholding or deduction for or on account of U.K. tax.

Income Tax

A dividend received by individual U.K. Holders will be subject to U.K. income tax. The system of grossing up dividends has been abolished from April 2016 and replaced with a simple rate of tax on dividends referred to below.

An individual holder of an ordinary share or ADS who is not a U.K. Holder will not be chargeable to U.K. income tax on a dividend paid by the company, unless such holder carries on (whether solely or in partnership) a trade, profession or vocation in the United Kingdom through a branch or agency in the United Kingdom to which the ordinary share or ADS is attributable. In these circumstances, such holder may, depending on his or her individual circumstances, be chargeable to U.K. income tax on a dividend received from the company.

The rate of U.K. income tax that is chargeable on dividends received in the tax year 2016/2017 onward by (i) an additional rate taxpayer is 38.1%, (ii) a higher rate taxpayer is 32.5%, and (iii) a basic rate taxpayer is 7.5%. An individual U.K. Holder may be entitled to a tax-free dividend allowance (in addition to their personal allowance) of £5,000 reduced to £2,000 for the tax year 2018/9. An individual's dividend income is treated as the top slice of their total income that is chargeable to U.K. income tax. Dividends which fall within an individual's personal income tax allowance do not form part of the dividend tax-free allowance.

Corporation Tax

A U.K. Holder within the charge to U.K. corporation tax may be entitled to exemption from U.K. corporation tax in respect of dividend payments. If the conditions for the exemption are not satisfied, or such U.K. Holder elects for an otherwise exempt dividend to be taxable, U.K. corporation tax will be chargeable on the gross amount of a dividend. If potential investors are in any doubt as to their position, they should consult their own professional advisers.

A corporate holder of an ordinary share or ADS that is not a U.K. Holder will not be subject to U.K. corporation tax on a dividend received from the company, unless it carries on a trade in the United Kingdom through a permanent establishment to which the ordinary share or ADS is attributable. In these circumstances, such holder may, depending on its individual circumstances and if the exemption from U.K. corporation tax discussed above does not apply, be chargeable to U.K. corporation tax on dividends received from the company.

Taxation of Disposals

U.K. Holders

A disposal or deemed disposal of an ordinary share or ADS by an individual U.K. Holder may, depending on his or her individual circumstances, give rise to a chargeable gain or to an allowable loss for the purpose of U.K. capital gains tax. The principal factors that will determine the capital gains tax position on a disposal of an ordinary share or ADS are the extent to which the holder realizes any other capital gains in the tax year in which the disposal is made, the extent to which the holder has incurred capital losses in that or any earlier tax year and the level of the annual exemption for tax-free gains in that tax year (the "annual exemption"). The annual exemption for the 2016/2017 tax year is £11,100 increasing to £11,300 for the 2017/18 tax year. If, after all allowable deductions, an individual U.K. Holder's total taxable income for the year exceeds the basic rate income tax limit, a taxable capital gain accruing on a disposal of an ordinary share or an ADS is taxed at the rate of 20%. In other cases, a taxable capital gain accruing on a disposal of an ordinary share or ADS may be taxed at the rate of 10% or the rate of 20% or at a combination of both rates.

An individual U.K. Holder who ceases to be resident in the United Kingdom (or who fails to be regarded as resident in a territory outside the United Kingdom for the purposes of double taxation relief) for a period of five tax years or less than five years and who disposes of an ordinary share or ADS during that period of temporary non-residence may be liable to U.K. capital gains tax on a chargeable gain accruing on such disposal on his or her return to the United Kingdom (or upon ceasing to be regarded as resident outside the United Kingdom for the purposes of double taxation relief) (subject to available exemptions or reliefs).

A disposal (or deemed disposal) of an ordinary share or ADS by a corporate U.K. Holder may give rise to a chargeable gain or an allowable loss for the purpose of U.K. corporation tax. Such a holder should be entitled to an indexation allowance, which applies to reduce a capital gain to the extent that such a gain arises due to inflation. The allowance may reduce a chargeable gain but will not create or increase an allowable loss.

Any gain or loss in respect of currency fluctuations over the period of holding an ordinary share or an ADS are also brought into account on a disposal.

Non-U.K. Holders

An individual holder who is not a U.K. Holder will not be liable to U.K. capital gains tax on capital gains realized on the disposal of an ordinary share or ADS unless such holder carries on (whether solely or in partnership) a trade, profession or vocation in the United Kingdom through a branch or agency in the United Kingdom to which the ordinary share or ADS is attributable. In these circumstances, such holder may, depending on his or her individual circumstances, be chargeable to U.K. capital gains tax on chargeable gains arising from a disposal of his or her ordinary share or ADS.

A corporate holder of an ordinary share or ADS that is not a U.K. Holder will not be liable for U.K. corporation tax on chargeable gains realized on the disposal of an ordinary share or ADS unless it carries on a trade in the United Kingdom through a permanent establishment to which the ordinary share or ADS is attributable. In these circumstances, a disposal (or deemed disposal) of an ordinary share or ADS by such holder may give rise to a chargeable gain or an allowable loss for the purposes of U.K. corporation tax.

Inheritance Tax

If for the purposes of the Double Taxation Relief (Taxes on Estates of Deceased Persons and on Gifts) Treaty United States of America Order 1979 (S1 1979/1454) between the United States and the United Kingdom an individual holder is domiciled at the time of their death or at the time of a transfer made during their lifetime, in the United States and is not a national of the United Kingdom, any ordinary share or ADS beneficially owned by that holder should not generally be subject to U.K. inheritance tax, provided that any applicable United States federal gift or estate tax liability is paid, except where (i) the ordinary share or ADS is part of the business property of a U.K. permanent establishment or pertain to a U.K. fixed base used for the performance of independent personal services; or (ii) the ordinary share or ADS is comprised in a settlement unless, at the time the settlement was made, the settlor was domiciled in the United States and not a national of the U.K. (in which case no charge to U.K. inheritance tax should apply).

Stamp Duty and Stamp Duty Reserve Tax

The stamp duty and stamp duty reserve tax, or SDRT, treatment of the issue and transfer of, and the agreement to transfer, an ordinary share outside a depositary receipt system or a clearance service are discussed in the paragraphs under "*General*" below. The stamp duty and SDRT treatment of such transactions in relation to such systems are discussed in the paragraphs under "*Depositary Receipt Systems and Clearance Services*" below.

General

An agreement to transfer an ordinary share will normally give rise to a charge to SDRT at the rate of 0.5% of the amount or value of the consideration payable for the transfer. SDRT is, in general, payable by the purchaser.

A transfer of an ordinary share or ADS will generally be subject to stamp duty at the rate of 0.5% of the consideration given for the transfer (rounded up to the next £5). The purchaser normally pays the stamp duty.

If a duly stamped transfer completing an agreement to transfer is produced within six years of the date on which the agreement is made (or, if the agreement is conditional, the date on which the agreement becomes unconditional) any SDRT already paid is generally repayable, normally with interest, and any SDRT charge yet to be paid is cancelled to avoid a double charge as the stamp duty has been paid.

Under current HMRC guidance, no U.K. stamp duty should be payable on a written instrument transferring an ADS or on a written agreement to transfer an ADS, on the basis that an ADS is not regarded as either "stock" or a "marketable security" for U.K. stamp duty purposes.

Depositary Receipt Systems and Clearance Services

Following the ECJ decision in C-569/07 *HSBC Holdings Plc, Vidacos Nominees Limited v The Commissioners of Her Majesty's Revenue & Customs* and the First-tier Tax Tribunal decision in *HSBC Holdings Plc and the Bank of New York Mellon Corporation v The Commissioners of Her Majesty's Revenue & Customs*, HM Revenue & Customs has confirmed that 1.5% SDRT is no longer payable when shares are issued or transferred to a clearance service (such as, in our understanding, DTC) or depositary receipt system as an integral part of a raising of capital.

Where an ordinary share or ADS is otherwise transferred (i) to, or to a nominee or an agent for, a person whose business is or includes the provision of clearance services or (ii) to, or to a nominee or an agent for a person whose business is or includes issuing depositary receipts, stamp duty or SDRT will generally be payable at the higher rate of 1.5% of the amount or value of the consideration given or, in certain circumstances, the value of the shares.

There is an exception from the 1.5% charge on the transfer to, or to a nominee or agent for, a clearance service where the clearance service has made and maintained an election under section 97A(1) of the Finance Act 1986, which has been approved by HM Revenue & Customs. In these circumstances, SDRT at the rate of 0.5% of the

amount or value of the consideration payable for the transfer will arise on any transfer of ordinary share into such an account and on subsequent agreements to transfer such shares within such account. It is our understanding that DTC has not made an election under section 97A(1) of the Finance Act of 1986.

Any liability for stamp duty or SDRT in respect of a transfer into a clearance service or depositary receipt system, or in respect of a transfer within such a service, which does arise will strictly be accountable by the clearance service or depositary receipt system operator or their nominee, as the case may be, but will, in practice, be payable by the participants in the clearance service or depositary receipt system.

The Proposed Financial Transactions Tax

The European Commission has published a proposal for a Directive for a common Financial Transactions Tax, or FTT, in Belgium, Germany, Greece, Spain, France, Italy, Austria, Portugal, Slovenia and Slovakia (described below as the "participating Member States").

The proposed FRR has very broad scope and could, if introduced in its current form, apply to certain dealings in ordinary shares (including secondary market transactions) in certain circumstances.

Under current proposals the FTT could apply in certain circumstances to persons both within and outside of the participating Member States. Generally, it would apply to certain dealings in ordinary shares where at least one party is a financial institution, and at least one party is established in a participating Member State. A financial institution may be, or be deemed to be, "established" in a participating Member State in a broad range of circumstance, including (i) by transacting with a person established in a participating Member State or (ii) where the financial instrument which is subject to the dealings is issued in participating Member State.

The FTT proposal remains subject to negotiation between the participating Member States. Further, the legality of the FTT proposals is at present uncertain. It may therefore be altered prior to any implementation, the timing of which remains unclear. Additional EU Member States may decide to participate. The FTT proposal remains only a proposal and little progress has been made in recent years; the impact of an FTT on us and holders of our ordinary shares and ADS is made more uncertain following the U.K.'s decision to withdraw from the European Union. Prospective holders of an ordinary share or ADS are advised to seek their own professional advice in relation to the FTT.

Taxation in the United States

The following summary of the material U.S. federal income tax consequences of the acquisition, ownership and disposition of the ADSs is based upon current law and does not purport to be a comprehensive discussion of all the tax considerations that may be relevant to a particular U.S. holder, as defined below, of the ADSs. This summary is based on current provisions of the Internal Revenue Code of 1986, as amended, or the Code, existing, final, temporary and proposed United States Treasury Regulations, administrative rulings and judicial decisions, in each case as available on the date of this Annual Report. All of the foregoing are subject to change, which change could apply retroactively and could affect the tax consequences described below.

This section summarizes the material U.S. federal income tax consequences to U.S. holders, as defined below, of an investment in the ADSs. This summary addresses only the U.S. federal income tax considerations for U.S. holders that acquire and hold the ADSs as capital assets. **Each prospective investor should consult a professional tax advisor with respect to the tax consequences of the acquisition, ownership or disposition of the ADSs**. This summary does not address tax considerations applicable to a holder of ADSs that may be subject to special tax rules including, without limitation, the following:

- banks or other financial institutions;
- insurance companies;
- dealers or traders in securities, currencies, or notional principal contracts;

- tax-exempt entities, including an "individual retirement account" or "Roth IRA" retirement plan;
- regulated investment companies or real estate investment trusts;
- persons that hold the ordinary shares as part of a hedge, straddle, conversion, constructive sale or similar transaction involving more than one position;
- an entity classified as a partnership and persons that hold the ordinary shares through partnerships or certain other pass-through entities;
- holders (whether individuals, corporations or partnerships) that are treated as expatriates for some or all U.S. federal income tax purposes;
- persons who acquired the ADSs as compensation for the performance of services;
- persons holding the ADSs in connection with a trade or business conducted outside of the United States;
- a U.S. holder who holds the ADSs through a financial account at a foreign financial institution that does not meet the requirements for avoiding withholding with respect to certain payments under Sections 1471 through 1474 of the Internal Revenue Code of 1986, as amended, or the Code;
- holders that own (or are deemed to own) 10% or more of our voting shares; and
- holders that have a "functional currency" other than the U.S. dollar.

Further, this summary does not address alternative minimum tax, gift or estate consequences or the indirect effects on the holders of equity interests in entities that own the ADSs. In addition, this discussion does not consider the U.S. tax consequences to holders of ADSs that are not "U.S. holders" (as defined below).

For the purposes of this summary, a "U.S. holder" is a beneficial owner of ordinary shares or ADSs that is (or is treated as), for U.S. federal income tax purposes:

- an individual who is either a citizen or resident of the United States;
- a corporation, or other entity that is treated as a corporation for U.S. federal income tax purposes, created or organized in or under the laws of the United States or any state of the United States or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust, if a court within the United States is able to exercise primary supervision over its administration and one or more U.S. persons have the authority to control all of the substantial decisions of such trust or has a valid election in effect under applicable U.S. Treasury Regulations to be treated as a United States person.

If a partnership holds ordinary shares or ADSs, the tax treatment of a partner will generally depend upon the status of the partner and upon the activities of the partnership.

We will not seek a ruling from the U.S. Internal Revenue Service, or IRS, with regard to the U.S. federal income tax treatment of an investment in our ordinary shares or ADSs, and we cannot assure you that the IRS will agree with the conclusions set forth below.

Ownership of ADSs

For U.S. federal income tax purposes, a holder of ADSs generally will be treated as the owner of the ordinary shares represented by such ADSs. Gain or loss will generally not be recognized on account of exchanges of ordinary shares for ADSs, or of ADSs for ordinary shares. References to ordinary shares in the discussion below are deemed to include ADSs, unless context otherwise requires.

Distributions

Subject to the discussion under "*Passive Foreign Investment Company Considerations*" below, the gross amount of any distribution actually or constructively received by a U.S. holder with respect to ordinary shares will be taxable to the U.S. holder as a dividend to the extent of such U.S. holder's pro rata share of our current and accumulated earnings and profits as determined under U.S. federal income tax principles. Distributions in excess of such pro rata share of our earnings and profits will be non-taxable to the U.S. holder to the extent of, and will be applied against and reduce, the U.S. holder's adjusted tax basis in the ordinary shares. Distributions in excess of the sum of such pro rata share of our earnings and profits and such adjusted tax basis will generally be taxable to the U.S. holder as capital gain from the sale or exchange of property. However, since we do not calculate our earnings and profits under U.S. federal income tax principles, it is expected that any distribution will be reported as a dividend, even if that distribution would otherwise be treated as a non-taxable return of capital or as capital gain under the rules described above. The amount of any distribution of property other than cash will be the fair market value of that property on the date of distribution. A corporate U.S. holder will not be eligible for any dividends-received deduction in respect of a dividend received with respect to ordinary shares.

Subject to the discussion below regarding the "Medicare tax," qualified dividends received by non-corporate U.S. holders (*i.e.*, individuals and certain trusts and estates) are currently subject to a maximum income tax rate of 20%. This reduced income tax rate is applicable to dividends paid by "qualified foreign corporations" to non-corporate U.S. holders that meet the applicable requirements, including a minimum holding period (generally, at least 61 days without protection from the risk of loss during the 121-day period beginning 60 days before the ex-dividend date). A non-United States corporation (other than a corporation that is classified as a PFIC for the taxable year in which the dividend is paid or the preceding taxable year) generally will be considered to be a qualified foreign corporation (a) if it is eligible for the benefits of a comprehensive tax treaty with the United States which the Secretary of Treasury of the United States determines is satisfactory for purposes of this provision and which includes an exchange of information provision, or (b) with respect to any dividend it pays on shares of stock which are readily tradable on an established securities market in the United States. Our ADSs are listed on the NASDAQ Global Market, which is an established securities market in the United States, and we expect the ADSs to be readily tradable on the NASDAQ Global Market. However, there can be no assurance that the ADSs will be considered readily tradable on an established securities market in the United States in later years. The Company, which is incorporated under the laws of the United Kingdom, believes that it qualifies as a resident of the United Kingdom for the purposes of, and is eligible for the benefits of, the Convention between the Government of the United States of America and the Government of the United Kingdom of Great Britain and Northern Ireland for the Avoidance of Double Taxation and the Prevention of Fiscal Evasion with Respect to Taxes on Income and Capital Gains, signed on July 24, 2001, or the U.S.-U.K. Tax Treaty, although there can be no assurance in this regard. Further, the IRS has determined that the U.S.-U.K. Tax Treaty is satisfactory for purposes of the qualified dividend rules and that it includes an exchange-of-information program. Based on the foregoing, we expect to be considered a qualified foreign corporation under the Code. Accordingly, dividends paid by us to non-corporate U.S. holders with respect to shares that meet the minimum holding period and other requirements are expected to be treated as "qualified dividend income." However, dividends paid by us will not qualify for the 20% maximum U.S. federal income tax rate if we are treated, for the tax year in which the dividends are paid or the preceding tax year, as a "passive foreign investment company" for U.S. federal income tax purposes, as discussed below.

The U.S. Treasury Department has announced its intention to issue rules regarding when and to what extent holders of ADSs will be permitted to rely on certifications from issuers to establish that dividends paid on shares to which such ADSs relate are treated as qualified dividends. Because such procedures have not yet been issued, it is not clear whether we will be able to comply with them.

Dividends received by a U.S. holder with respect to ordinary shares generally will be treated as foreign source income for the purposes of calculating that holder's foreign tax credit limitation. For these purposes, dividends distributed by us generally will constitute "passive category income" (but, in the case of some U.S. holders, may constitute "general category income").

Sale or Other Disposition of Ordinary Shares

A U.S. holder will generally recognize gain or loss for U.S. federal income tax purposes upon the sale or exchange of ordinary shares in an amount equal to the difference between the U.S. dollar value of the amount realized from such sale or exchange and the U.S. holder's tax basis for those ordinary shares. Subject to the discussion under "*Passive Foreign Investment Company Considerations*" below, this gain or loss will generally be a capital gain or loss and will generally be treated as from sources within the United States. Such capital gain or loss will be treated as long-term capital gain or loss if the U.S. holder has held the ordinary shares for more than one year at the time of the sale or exchange. Long-term capital gains of non-corporate U.S. holders may be eligible for a preferential tax rate; the deductibility of capital losses is subject to limitations. For a cash basis taxpayer, units of foreign currency paid or received are translated into U.S. dollars at the spot rate on the settlement date of the purchase or sale. In that case, no foreign currency exchange gain or loss will result from currency fluctuations between the trade date and the settlement date of such a purchase or sale. An accrual basis taxpayer, however, may elect the same treatment required of cash basis taxpayers with respect to purchases and sales of the ADSs that are traded on an established securities market, provided the election is applied consistently from year to year. Such election may not be changed without the consent of the IRS. For an accrual basis taxpayer who does not make such election, units of foreign currency paid or received are translated into U.S. dollars at the spot rate on the trade date of the purchase or sale. Such an accrual basis taxpayer may recognize exchange gain or loss based on currency fluctuations between the trade date and settlement date. Any foreign currency gain or loss a U.S. holder realizes will be U.S. source ordinary income or loss.

Medicare Tax

An additional 3.8% tax, or "Medicare Tax", is imposed on all or a portion of the "net investment income" (which includes taxable dividends and net capital gains, adjusted for deductions properly allocable to such dividends or net capital gains) received by (i) U.S. holders that are individuals with modified adjusted gross income of over $200,000 ($250,000 in the case of joint filers, $125,000 in the case of married individuals filing separately) and (ii) certain trusts or estates.

Passive Foreign Investment Company Considerations

A corporation organized outside the United States generally will be classified as a passive foreign investment company, or PFIC, for U.S. federal income tax purposes in any taxable year in which, after applying the applicable look-through rules, either: (i) at least 75% of its gross income is passive income, or (ii) on average at least 50% of the gross value of its assets is attributable to assets that produce passive income or are held for the production of passive income. In arriving at this calculation, a pro rata portion of the income and assets of each corporation in which we own, directly or indirectly, at least a 25% interest, as determined by the value of such corporation, must be taken into account. Passive income for this purpose generally includes dividends, interest, royalties, rents and gains from commodities and securities transactions.

We believe that we were not a PFIC for any previous taxable year. Based on our estimated gross income, the average value of our gross assets, and the nature of the active businesses conducted by our "25% or greater" owned subsidiaries, we do not believe that we will be classified as a PFIC in the current taxable year. Our status for any taxable year will depend on our assets and activities in each year, and because this is a factual determination made annually after the end of each taxable year, there can be no assurance that we will not be considered a PFIC for the current taxable year or any future taxable year. The market value of our assets may be determined in large part by reference to the market price of the ADSs and our ordinary shares, which is likely to fluctuate after the offering (and may fluctuate considerably given that market prices of life sciences companies can be especially volatile). In addition, the composition of our income and assets will be affected by how, and how quickly, we spend the cash we raise in this offering.

If we were a PFIC for any taxable year during which a U.S. holder held ordinary shares, under the "default PFIC regime" (i.e., in the absence of one of the elections described below) gain recognized by the U.S. holder on a sale

or other disposition (including a pledge) of the ordinary shares would be allocated ratably over the U.S. holder's holding period for the ordinary shares. The amounts allocated to the taxable year of the sale or other disposition and to any year before we became a PFIC would be taxed as ordinary income. The amount allocated to each other taxable year would be subject to tax at the highest rate in effect for individuals or corporations, as appropriate, for that taxable year, and an interest charge would be imposed on the resulting tax liability for that taxable year. Similar rules would apply to the extent any distribution in respect of ordinary shares exceeds 125% of the average of the annual distributions on ordinary shares received by a U.S. holder during the preceding three years or the holder's holding period, whichever is shorter.

In the event we were treated as a PFIC, the tax consequences under the default PFIC regime described above could be avoided by either a "mark-to-market" or "qualified electing fund" election. A U.S. holder making a mark-to-market election (if the eligibility requirements for such an election were satisfied) generally would not be subject to the PFIC rules discussed above, except with respect to any portion of the holder's holding period that preceded the effective date of the election. Instead, the electing holder would include in ordinary income, for each taxable year in which we were a PFIC, an amount equal to any excess of (a) the fair market value of the ordinary shares as of the close of such taxable year over (b) the electing holder's adjusted tax basis in such ordinary shares. In addition, an electing holder would be allowed a deduction in an amount equal to the lesser of (a) the excess, if any, of (i) the electing holder's adjusted tax basis in the ordinary shares over (ii) the fair market value of such ordinary shares as of the close of such taxable year or (b) the excess, if any, of (i) the amount included in ordinary income because of the election for prior taxable years over (ii) the amount allowed as a deduction because of the election for prior taxable years. The election would cause adjustments in the electing holder's tax basis in the ordinary shares to reflect the amount included in gross income or allowed as a deduction because of the election. In addition, upon a sale or other taxable disposition of ordinary shares, an electing holder would recognize ordinary income or loss (not to exceed the excess, if any, of (a) the amount included in ordinary income because of the election for prior taxable years over (b) the amount allowed as a deduction because of the election for prior taxable years).

Alternatively, a U.S. holder making a valid and timely "QEF election" generally would not be subject to the default PFIC regime discussed above. Instead, for each PFIC year to which such an election applied, the electing holder would be subject to U.S. federal income tax on the electing holder's pro rata share of our net capital gain and ordinary earnings, regardless of whether such amounts were actually distributed to the electing holder. However, because we do not intend to prepare or provide the information that would permit the making of a valid QEF election, that election will not be available to U.S. holders.

If we were considered a PFIC for the current taxable year or any future taxable year, a U.S. holder would be required to file annual information returns for such year, whether or not the U.S. holder disposed of any ordinary shares or received any distributions in respect of ordinary shares during such year.

Backup Withholding and Information Reporting

U.S. holders generally will be subject to information reporting requirements with respect to dividends on ordinary shares and on the proceeds from the sale, exchange or disposition of ordinary shares that are paid within the United States or through U.S.-related financial intermediaries, unless the U.S. holder is an "exempt recipient." In addition, U.S. holders may be subject to backup withholding (at a 28% rate) on such payments, unless the U.S. holder provides a taxpayer identification number and a duly executed IRS Form W-9 or otherwise establishes an exemption. Backup withholding is not an additional tax, and the amount of any backup withholding will be allowed as a credit against a U.S. holder's U.S. federal income tax liability and may entitle such holder to a refund, provided that the required information is timely furnished to the IRS.

Foreign Account Tax Compliance Act, or FATCA, and Related Provisions

Under certain circumstances, the Company or its paying agent may be required, pursuant to the FATCA provisions of the Code (or analogous provisions of non-U.S. law) and regulations or pronouncements

thereunder, any "intergovernmental agreement" entered into pursuant to those provisions or any U.S. or non-U.S. fiscal or regulatory legislation, rules, guidance notes or practices adopted pursuant to any such agreement, to withhold U.S. tax at a rate of 30% on all or a portion of payments of dividends or other corporate distributions which are treated as "foreign passthru payments" made on or after the earlier of (i) January 1, 2019 and (ii) the date of publication of final regulations defining the term "foreign passthru payment," if such payments are not exempt from such withholding. The Company believes, and this discussion assumes, that the Company is not a "foreign financial institution" for purposes of FATCA. The rules regarding FATCA and "foreign pass-thru payments," including the treatment of proceeds from the disposition of ordinary shares, are not completely clear, and further guidance may be issued by the IRS that would clarify how FATCA might apply to dividends or other amounts paid on or with respect to ordinary shares.

Foreign Asset Reporting

In addition, certain individuals who are U.S. Holders may be required to file IRS Form 8938 to report the ownership of "specified foreign financial assets" if the total value of those assets exceeds an applicable threshold amount (subject to certain exceptions). For these purposes, a specified foreign financial asset may include not only a financial account (as defined for these purposes) maintained by a non-U.S. financial institution, but also stock or securities issued by a non-U.S. corporation (such as the Company). Certain U.S. entities may also be required to file IRS Form 8938 in the future.

F. Dividends and Paying Agents

Not applicable.

G. Statement by Experts

Not applicable.

H. Documents on Display

We are subject to the informational requirements of the Exchange Act. Accordingly, we are required to file reports and other information with the SEC, including annual reports on Form 20-F and reports on Form 6-K. You may inspect and copy reports and other information filed with the SEC at the Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549. Information on the operation of the Public Reference Room may be obtained by calling the SEC at 1-800-SEC-0330. In addition, the SEC maintains an Internet website that contains reports and other information about issuers, like us, that file electronically with the SEC. The address of that website is www.sec.gov.

We also make available on our website, free of charge, our Annual Report and the text of our reports on Form 6-K, including any amendments to these reports, as well as certain other SEC filings, as soon as reasonably practicable after they are electronically filed with or furnished to the SEC. Our website address is "www.summitplc.com." The information contained on our website is not incorporated by reference in this Annual Report.

Item 11: Quantitative and Qualitative Disclosures About Market Risk

Our activities expose us to a variety of financial risks: foreign currency risk, interest rate risk, credit risk and liquidity risk. Our principal financial instrument comprises cash and cash equivalents, and this is used to finance our operations. We have various other financial instruments such as trade receivables and payables that arise directly from our operations. The category of loans and receivables contains only trade and other receivables, shown on the face of the balance sheet, all of which mature within one year. We have compared fair value to book value for each class of financial asset and liability and no difference was identified, other than in respect of

financial liabilities on funding arrangements. The fair value at January 31, 2017 was calculated to be £8.3 million. Further information is included in note 18 to our consolidated financial statements appearing at the end of this Annual Report. We have a policy, which has been consistently followed, of not trading in financial instruments.

Foreign Currency Risk

Foreign currency risk refers to the risk that the value of a financial commitment or recognized asset or liability will fluctuate due to changes in foreign currency rates. Our net income and financial position, as expressed in pounds sterling, are exposed to movements in foreign exchange rates against the U.S. dollar and the euro. The main trading currencies are pounds sterling, the U.S. dollar, and the euro. We are exposed to foreign currency risk as a result of operating transactions, capital raises in the United States, payment in U.S. dollars for any milestone or royalty payment received under our license and collaboration agreement with Sarepta and the translation for foreign bank accounts. We monitor our exposure to foreign exchange risk. Exposures are generally managed through natural hedging via the currency denomination of cash balances and any impact currently is not material to us.

Interest Rate Risk

We do not hold any derivative instruments to manage interest rate risk.

Credit Risk

Our credit risk with respect to customers is limited and we did not have any trade receivables outstanding as of January 31, 2017. Financial instruments that potentially expose us to concentrations of credit risk consist primarily of short-term cash investments and trade accounts receivable.

Liquidity Risk

We have funded our operations since inception primarily through the issuance of equity securities. We have also received funding from our license and collaboration agreement with Sarepta, as well as philanthropic, non-government and not for profit organizations and patient advocacy groups and grant funding from government entities. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of public or private equity or debt financings or other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Our inability to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

Item 12: Description of Securities other than Equity Securities

A. Debt Securities.

Not applicable.

B. Warrants and Rights.

Not applicable.

C. Other Securities.

Not applicable.

D. American Depositary Shares.

Fees and Expenses

The following table shows the fees and charges that a holder of our ADSs may have to pay, either directly or indirectly. The majority of these costs are set by the depositary and are subject to change:

Persons depositing or withdrawing shares or ADS holders must pay:	For:
$5.00 (or less) per 100 ADSs (or portion of 100 ADSs)	Issuance of ADSs, including issuances resulting from a distribution of shares or rights or other property Cancellation of ADSs for the purpose of withdrawal, including if the deposit agreement terminates
$.05 (or less) per ADS	Any cash distribution to ADS holders
A fee equivalent to the fee that would be payable if securities distributed to you had been shares and the shares had been deposited for issuance of ADSs	Distribution of securities distributed to holders of deposited securities (including rights) that are distributed by the depositary to ADS holders
$.05 (or less) per ADS per calendar year	Depositary services
Registration or transfer fees	Transfer and registration of shares on our share register to or from the name of the depositary or its agent when you deposit or withdraw shares
Expenses of the depositary	Cable, telex and facsimile transmissions (when expressly provided in the deposit agreement) Converting foreign currency to U.S. dollars
Taxes and other governmental charges the depositary or the custodian has to pay on any ADSs or shares underlying ADSs, such as stock transfer taxes, stamp duty or withholding taxes	As necessary
Any charges incurred by the depositary or its agents for servicing the deposited securities	As necessary

The depositary collects its fees for delivery and surrender of ADSs directly from investors depositing shares or surrendering ADSs for the purpose of withdrawal or from intermediaries acting for them. The depositary collects fees for making distributions to investors by deducting those fees from the amounts distributed or by selling a portion of distributable property to pay the fees. The depositary may collect its annual fee for depositary services by deduction from cash distributions or by directly billing investors or by charging the book-entry system accounts of participants acting for them. The depositary may collect any of its fees by deduction from any cash distribution payable (or by selling a portion of securities or other property distributable) to ADS holders that are obligated to pay those fees. The depositary may generally refuse to provide fee-attracting services until its fees for those services are paid.

From time to time, the depositary may make payments to us to reimburse us for costs and expenses generally arising out of establishment and maintenance of the ADS program, waive fees and expenses for services provided to us by the depositary or share revenue from the fees collected from ADS holders. In performing its duties under the deposit agreement, the depositary may use brokers, dealers or other service providers that are affiliates of the depositary and that may earn or share fees or commissions.

Payment of Taxes

You will be responsible for any taxes or other governmental charges payable on your ADSs or on the deposited securities represented by any of your ADSs. The depositary may refuse to register any transfer of your ADSs or allow you to withdraw the deposited securities represented by your ADSs until those taxes or other charges are paid. It may apply payments owed to you or sell deposited securities represented by your American Depositary Shares to pay any taxes owed and you will remain liable for any deficiency. If the depositary sells deposited securities, it will, if appropriate, reduce the number of ADSs to reflect the sale and pay to ADS holders any proceeds, or send to ADS holders any property, remaining after it has paid the taxes.

PART II

Item 13: Defaults, Dividend Arrearages and Delinquencies

None.

Item 14: Material Modifications to the Rights of Security Holders and Use of Proceeds

A. Not applicable.

B. Not applicable.

C. Not applicable.

D. Not applicable.

E. Use of Proceeds

We effected the initial public offering, or IPO, of our American Depositary Shares, or ADSs, each representing five of our ordinary shares, through a Registration Statement on Form F-1 (File No. 333-201807) that was declared effective by the Securities and Exchange Commission on March 4, 2015. On March 10, 2015, we completed the sale of 3,450,000 ADSs in our IPO at a price to the public of $9.90 per share, resulting in net proceeds to us of $28.0 million after deducting underwriting discounts and commissions of $2.4 million and offering expenses of $3.8 million. In addition, we granted the underwriters a 30-day option to purchase up to 517,500 additional ADSs to cover over allotments, at the public offering price, less the underwriting discount. On March 18, 2015, we completed the additional sale of 517,500 ADSs under this option at a price to the public of $9.90 per share, resulting in net proceeds to us of $4.8 million after deducting underwriting discounts and commissions of $0.4 million. The offering commenced on March 4, 2015 and did not terminate until we had completed the sale of all of the securities registered in the offering. None of the underwriting discounts and commissions or other offering expenses were paid to directors or officers of ours or their associates or to persons owning 10% or more of any class of our equity securities or to any affiliates of ours. JMP Securities LLC, Oppenheimer & Co. Inc. and Needham & Company, LLC were the underwriters for our IPO.

We received proceeds of approximately $32.7 million from our IPO, net of underwriting discounts and commissions and the expenses described above. As of January 31, 2017, we had used all of the net proceeds from the IPO, as follows:

- approximately $21.6 million related to our DMD activities including to complete our Phase 1b modified diet clinical trial, to complete our Phase 1 clinical trial that evaluated a new formulation of ezutromid, to advance our ongoing PhaseOut DMD clinical trial and to advance our pipeline of future generation utrophin modulators;
- approximately $6.7 million to complete our Phase 2 clinical trial of ridinilazole and advance our ongoing exploratory Phase 2 clinical trial of ridinilazole compared to fidaxomicin;
- approximately $2.0 million to prepare ridinilazole for our two, planned Phase 3 clinical trials; and
- approximately $2.4 million on working capital and general corporate purposes.

Pending such expenditures, we deposited such proceeds with reputable U.K.-based and U.S.-based banking institutions.

Item 15: Controls and Procedures.

A. Disclosure Controls and Procedures.

We have carried out an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures (as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) under the supervision and the participation of the company's management, which is responsible for the management of the internal controls, and which includes our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively). The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Securities Exchange Act of 1934 is recorded, processed, summarized and reported within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Securities Exchange Act of 1934 is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. There are inherent limitations to the effectiveness of any system of disclosure controls and procedures, including the possibility of human error and the circumvention or overriding of the controls and procedures. Accordingly, even effective disclosure controls and procedures can only provide reasonable assurance of achieving their control objectives. Based upon our evaluation of our disclosure controls and procedures as of January 31, 2017, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at a reasonable level of assurance.

B. Management's Annual Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Our internal control over financial reporting is a process designed, under the supervision of the Chief Executive Officer and the Chief Financial Officer, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of our financial statements for external reporting purposes in accordance with International Financial Reporting Standards.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect all misstatements. Moreover, projections of any evaluation of the effectiveness of internal control to future periods are subject to a risk that controls may become inadequate because of changes in conditions and that the degree of compliance with the policies or procedures may deteriorate.

Our management has assessed the effectiveness of internal control over financial reporting as of January 31, 2017 based on the Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) 2013. Based on this assessment, our management has concluded that our internal control over financial reporting as of January 31, 2017 was effective.

C. Attestation Report of the Registered Public Accounting Firm

This report does not include an attestation report of our registered public accounting firm as we are an emerging growth company.

D. Changes in Internal Control Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the fiscal year ended January 31, 2017 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 16A: Audit Committee Financial Expert

The members of our audit committee are Mr. Wurzer, Dr. Price and Ms. Andrews. Mr. Wurzer is the chair of the audit committee. Each of our audit committee members satisfies the independence requirements of Rule 5605(a)(2) of the NASDAQ Stock Market Marketplace Rules and the independence requirements of Rule 10A-3(b)(1) under the Exchange Act. Our board of directors has determined that Mr. Wurzer is an "audit committee financial expert" as defined in Item 16A of Form 20-F.

Item 16B: Code of Ethics

Our Code of Business Conduct and Ethics is applicable to all of our employees, officers and directors and is available on our website at http://www.summitplc.com. Our Code of Business Conduct and Ethics provides that our directors and officers are expected to avoid any action, position or interest that conflicts with the interests of our company or gives the appearance of a conflict. Our directors and officers have an obligation under our Code of Business Conduct and Ethics to advance our company's interests when the opportunity to do so arises. We expect that any amendment to this code, or any waivers of its requirements, will be disclosed on our website. Information contained on, or that can be accessed through, our website is not incorporated by reference into this Annual Report, and you should not consider information on our website to be part of this Annual Report.

Item 16C: Principal Accountant Fees and Services

The following table sets forth, for each of the years indicated, the aggregate fees billed to us for services rendered by PricewaterhouseCoopers LLP, our independent registered public accounting firm.

	Year Ended January 31,	
	2017	2016
	(in thousands)	
Audit Fees	£ 230	£ 115
Audit-Related Fees(1)	166	164
Tax Fees(2)	62	20
All Other Fees(3)	—	—
Total	£ 458	£ 299

(1) For the year ended January 31, 2017, audit-related fees includes assurance reporting on information included in our registration statement on Form F-3 that was originally filed with the U.S. Securities and Exchange Commission on May 12, 2016.
(2) Fees relate to the aggregated fees for services rendered on tax compliance, tax advice and tax planning.
(3) No fees incurred in this category.

Item 16D: Exemptions from the Listing Standards for Audit Committees

Not applicable.

Item 16E: Purchases of Equity Securities by the Issuer and Affiliated Purchasers.

Not applicable.

Item 16F: Change in Registrant's Certifying Accountant

Not applicable.

Item 16G: Corporate Governance

The Sarbanes-Oxley Act of 2002, as well as related rules subsequently implemented by the SEC, requires foreign private issuers, including our company, to comply with various corporate governance practices. In addition, NASDAQ rules provide that foreign private issuers may follow home country practice in lieu of the NASDAQ corporate governance standards, subject to certain exceptions and except to the extent that such exemptions would be contrary to U.S. federal securities laws. The home country practices followed by our company in lieu of NASDAQ rules are described below:

- We do not follow NASDAQ's quorum requirements applicable to meetings of shareholders. Such quorum requirements are not required under U.K. law. In accordance with generally accepted business practice, our articles of association provide alternative quorum requirements that are generally applicable to meetings of shareholders.
- We do not follow NASDAQ's requirements that non-management directors meet on a regular basis without management present. Our board of directors may choose to meet in executive session at their discretion.
- We do not follow NASDAQ's requirements to seek shareholder approval for the implementation of certain equity compensation plans, the issuances of ordinary shares under such plans, or in connection with certain private placements of equity securities. In accordance with U.K. law, we are not required to seek shareholder approval to allot ordinary shares in connection with applicable employee equity compensation plans. We will follow U.K. law with respect to any requirement to obtain shareholder approval prior to any private placements of equity securities.

We intend to take all actions necessary for us to maintain compliance as a foreign private issuer under the applicable corporate governance requirements of the Sarbanes-Oxley Act of 2002, the rules adopted by the SEC and NASDAQ's listing standards.

Because we are a foreign private issuer, our directors and senior management are not subject to short-swing profit and insider trading reporting obligations under Section 16 of the U.S. Securities Exchange Act of 1934, as amended, or Exchange Act. They are, however, subject to the obligations to report changes in share ownership under Section 13 of the Exchange Act and related SEC rules.

Item 16H: Mine Safety Disclosure

Not applicable.

PART III

Item 17: Financial Statements

We have elected to provide financial statements pursuant to Item 18.

Item 18: Financial Statements

The financial statements are filed as part of this Annual Report beginning on page F-1.

Item 19: Exhibits

Exhibit No.	Description
1.1	Articles of Association of Summit Therapeutics plc (incorporated by reference to Exhibit 3.1 to the Company's Registration Statement on Form F-1 (File No. 333-201807), as amended, filed with the Securities and Exchange Commission on February 20, 2015)
2.1	Specimen certificate evidencing ordinary shares of Summit Therapeutics plc (incorporated by reference to Exhibit 4.5 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
2.2	Form of Deposit Agreement among Summit Therapeutics plc, The Bank of New York Mellon, as depositary, and all Owners and Holders of ADSs issued thereunder (incorporated by reference to Exhibit 4.1 to the Company's Registration Statement on Form F-1 (File No. 333-201807), as amended, filed with the Securities and Exchange Commission on February 20, 2015)
2.3	Form of American Depositary Receipt (included in Exhibit 2.2) (incorporated by reference to Exhibit 4.2 to the Company's Registration Statement on Form F-1 (File No. 333-201807), as amended, filed with the Securities and Exchange Commission on February 20, 2015)
2.4	Warrant Instrument, dated April 4, 2012, creating warrant to subscribe for shares in Summit Therapeutics plc issued to Singer Capital Markets Limited (incorporated by reference to Exhibit 4.3 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
2.5	Warrant Instrument, dated November 22, 2013, relating to Warrants in Registered Form to Subscribe for Ordinary Shares in Summit Therapeutics plc (incorporated by reference to Exhibit 4.4 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.1†	Grant Agreement, entered into as of December 15, 2011, by and between Duchenne Partners Fund and Summit Therapeutics plc (incorporated by reference to Exhibit 10.1 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.2†	MDA Venture Philanthropy Grant Contract, entered into as of December 15, 2011, by and between Muscular Dystrophy Association, Inc. and Summit Therapeutics plc (incorporated by reference to Exhibit 10.2 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.3†	Translation Award Funding Agreement, entered into as of October 19, 2012, by and between the Wellcome Trust Limited and Summit Therapeutics plc (incorporated by reference to Exhibit 10.3 to the Company's Registration Statement on Form F-1 (File No. 333-201807), as amended, filed with the Securities and Exchange Commission on February 27, 2015)
4.4†	Agreement for the Sponsorship of a Research Programme, dated November 22, 2013, by and between The Chancellor Masters and Scholars of the University of Oxford; Isis Innovation Limited; and Summit Therapeutics plc (incorporated by reference to Exhibit 10.4 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.5†	Deed of Licence of Know-How, dated November 22, 2013, by and between Isis Innovation Limited and MuOx Limited (incorporated by reference to Exhibit 10.5 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)

Exhibit No.	Description
4.6†	Supplemental Variation Deed, dated July 24, 2014, by and between Isis Innovation Limited and MuOx Limited (incorporated by reference to Exhibit 10.6 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.7†	Option Agreement, dated November 22, 2013, by and between The Chancellor Masters and Scholars of the University of Oxford, Isis Innovation Limited and Summit Therapeutics plc (incorporated by reference to Exhibit 10.7 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.8†	Variation Agreement, dated July 16, 2014, by and between The Chancellor Masters and Scholars of the University of Oxford, Isis Innovation Limited and Summit Therapeutics plc (incorporated by reference to Exhibit 10.8 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.9	Lease, dated June 21, 2013, by and between MEPC Milton Park No. 1 Limited and MEPC Milton Park No. 2 Limited on behalf of MEPC Milton LP and Summit Therapeutics plc (incorporated by reference to Exhibit 10.9 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.10	Service Agreement, effective as of January 14, 2015, by and between Cambridge Innovation Center and Summit Therapeutics Inc. (incorporated by reference to Exhibit 10.10 to the Company's Registration Statement on Form F-1 (File No. 333-201807), as amended, filed with the Securities and Exchange Commission on February 20, 2015)
4.11	2005 Enterprise Management Incentive Scheme (incorporated by reference to Exhibit 10.11 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.12	Letter of Appointment, dated November 20, 2014, by and between Summit Therapeutics Inc. and Valerie Andrews (incorporated by reference to Exhibit 10.12 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.13	Letter of Appointment, dated November 21, 2012, by and between Summit Therapeutics plc and Frank Armstrong (incorporated by reference to Exhibit 10.13 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.14	Letter of Appointment, dated December 19, 2013, by and between Summit Therapeutics plc and Stephen Davies (incorporated by reference to Exhibit 10.14 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.15	Letter of Appointment, dated August 8, 2013, by and between Summit Therapeutics plc and Barry Price (incorporated by reference to Exhibit 10.15 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)
4.16	Letter of Appointment, dated April 16, 2014, by and between Summit Therapeutics plc and Leopoldo Zambeletti (incorporated by reference to Exhibit 10.16 to the Company's Registration Statement on Form F-1 (File No. 333-201807), filed with the Securities and Exchange Commission on January 30, 2015)

Exhibit No.	Description
4.17	Letter of Appointment, dated February 18, 2015, by and between Summit Therapeutics plc and David Wurzer (incorporated by reference to Exhibit 10.17 to the Company's Registration Statement on Form F-1 (File No. 333-201807), as amended, filed with the Securities and Exchange Commission on February 20, 2015)
4.18	Form of Deed of Indemnity (incorporated by reference to Exhibit 10.18 to the Company's Registration Statement on Form F-1 (File No. 333-201807), as amended, filed with the Securities and Exchange Commission on February 20, 2015)
4.19	Deed of Variation, dated November 16, 2015, relating to the Warrant Instrument, dated November 22, 2013 (incorporated by reference to Exhibit 4.19 to the Company's Annual Report on Form 20-F (File No. 001-36866), filed with the Securities and Exchange Commission on May 12, 2016)
4.20†	Variation Agreement, dated November 16, 2015, relating to the Option Agreement, dated November 22, 2013, by and between the University of Oxford, Isis Innovation Limited and Summit Therapeutics plc (incorporated by reference to Exhibit 4.20 to the Company's Annual Report on Form 20-F (File No. 001-36866), filed with the Securities and Exchange Commission on May 12, 2016)
4.21†	Second Variation Agreement, dated November 16, 2015, relating to the Agreement for the Sponsorship of a Research Programme, dated November 22, 2013, by and between the Chancellor Masters and Scholars of the University of Oxford, Isis Innovation Limited and Summit Therapeutics plc (incorporated by reference to Exhibit 4.21 to the Company's Annual Report on Form 20-F (File No. 001-36866), filed with the Securities and Exchange Commission on May 12, 2016)
4.22	2016 Long Term Incentive Plan (incorporated by reference to Exhibit 4.22 to the Company's Annual Report on Form 20-F (File No. 001-36866), filed with the Securities and Exchange Commission on May 12, 2016)
4.23*+	License and Collaboration Agreement, dated October 3, 2016, by and between Summit (Oxford) Ltd. and Sarepta Therapeutics, Inc.
4.24*+	Deed of Novation and Variation, dated March 3, 2017, among MuOx Limited, Oxford University Innovation Limited (formerly Isis Innovation Limited) and Summit (Oxford) Limited
4.25*	Lease, dated February 17, 2017, by and among MEPC Milton Park No. 1 Limited, MEPC Milton Park No. 2 Limited and Summit Therapeutics plc
8.1*	Subsidiaries of Summit Therapeutics plc
12.1*	Certification of Chief Executive Officer pursuant to Securities Exchange Act Rules 13a-14(a) and 15d-14(a) as adopted pursuant to §302 of the Sarbanes-Oxley Act of 2002
12.2*	Certification of Chief Financial Officer pursuant to Securities Exchange Act Rules 13a-14(a) and 15d-14(a) as adopted pursuant to §302 of the Sarbanes-Oxley Act of 2002
13.1*	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002
15.1*	Consent of PricewaterhouseCoopers LLP

* Filed herewith.
† Confidential treatment has been granted as to certain portions of the exhibit. Confidential materials omitted and filed separately with the Securities and Exchange Commission.
+ Confidential treatment has been requested as to certain portions of the exhibit. Confidential materials omitted and filed separately with the Securities and Exchange Commission.

SIGNATURES

The registrant hereby certifies that it meets all of the requirements for filing on Form 20-F and that it has duly caused and authorized the undersigned to sign this Annual Report on its behalf.

SUMMIT THERAPEUTICS PLC

By: /s/ Glyn Edwards
Name: Glyn Edwards
Title: Chief Executive Officer

Date: March 30, 2017

SUMMIT THERAPEUTICS PLC

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Report of Independent Registered Public Accounting Firm

To the board of directors and shareholders of Summit Therapeutics plc:

In our opinion, the accompanying consolidated statement of financial position and the related consolidated statements of comprehensive income, cash flows, and changes in equity present fairly, in all material respects, the financial position of Summit Therapeutics plc and its subsidiaries at January 31, 2017 and January 31, 2016, and the results of their operations and their cash flows for each of the three years in the period ended January 31, 2017 in conformity with International Financial Reporting Standards as issued by the International Accounting Standards Board. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits of these statements in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

PricewaterhouseCoopers LLP
Reading, United Kingdom

March 29, 2017

Consolidated Statement of Financial Position
At January 31, 2017 and 2016 and February 1, 2015

	Note	**January 31, 2017 £000**	January 31, 2016 Adjusted* £000	February 1, 2015 Adjusted* £000
ASSETS				
Non-current assets				
Goodwill	11	**664**	664	664
Intangible assets	12	**3,470**	3,473	3,483
Property, plant and equipment	13	**116**	83	55
		4,250	4,220	4,202
Current assets				
Prepayments and other receivables	14	**1,027**	1,519	2,630
Current tax receivable		**4,248**	3,014	1,299
Cash and cash equivalents		**28,062**	16,304	11,265
		33,337	20,837	15,194
Total assets		**37,587**	25,057	19,396
LIABILITIES				
Non-current liabilities				
Deferred income	16	**(23,615)**	—	—
Financial liabilities on funding arrangements	17	**(5,919)**	(5,034)	(2,155)
Provisions for other liabilities and charges	19	**(85)**	(73)	(45)
Deferred tax liability	20	**(565)**	(664)	(664)
		(30,184)	(5,771)	(2,864)
Current liabilities				
Trade and other payables	15	**(3,984)**	(3,206)	(3,570)
Deferred income	16	**(6,912)**	—	—
		(10,896)	(3,206)	(3,570)
Total liabilities		**(41,080)**	(8,977)	(6,434)
Net (liabilities) / assets		**(3,493)**	16,080	12,962
EQUITY				
Share capital	21	**618**	613	411
Share premium account		**46,420**	46,035	24,101
Share-based payment reserve		**5,136**	3,757	2,597
Merger reserve		**(1,943)**	(1,943)	(1,943)
Special reserve		**19,993**	19,993	19,993
Currency translation reserve		**50**	21	62
Accumulated losses reserve		**(73,767)**	(52,396)	(32,259)
Total (deficit) / equity		**(3,493)**	16,080	12,962

* See Note 1 ‘Change in accounting policy’

The accompanying notes form an integral part of these Consolidated Financial Statements.

Consolidated Statement of Comprehensive Income
For the year ended January 31, 2017, 2016 and 2015

	Note	**Year ended January 31, 2017** **£000**	Year ended January 31, 2016 Adjusted* £000	Year ended January 31, 2015 Adjusted* £000
Revenue	5	**2,304**	—	—
Other operating income	7	**72**	1,281	1,888
Operating expenses				
Research and development	7	**(18,952)**	(16,856)	(10,417)
General and administration	7	**(8,277)**	(4,771)	(3,704)
Total operating expenses		**(27,229)**	(21,627)	(14,121)
Operating loss		**(24,853)**	(20,346)	(12,233)
Finance income		**8**	30	51
Finance cost	17	**(862)**	(2,879)	(499)
Loss before income tax		**(25,707)**	(23,195)	(12,681)
Income tax	9	**4,336**	3,058	1,297
Loss for the year		**(21,371)**	(20,137)	(11,384)
Other comprehensive income / (loss)				
Exchange differences on translating foreign operations		**29**	(41)	62
Total comprehensive loss		**(21,342)**	(20,178)	(11,322)
Basic and diluted earnings per Ordinary Share from operations	10	**(35)p**	**(34)p**	**(29)p**

* See Note 1 ‘Change in accounting policy’

The accompanying notes form an integral part of these Consolidated Financial Statements.

Consolidated Statement of Cash Flows
For the year ended January 31, 2017, 2016 and 2015

	Note	**Year ended January 31, 2017 £000s**	Year ended January 31, 2016 Adjusted* £000s	Year ended January 31, 2015 Adjusted* £000s
Cash flows from operating activities				
Loss before income tax		**(25,707)**	(23,195)	(12,681)
		(25,707)	(23,195)	(12,681)
Adjusted for:				
Finance income		**(8)**	(30)	(51)
Finance cost		**862**	2,879	499
Foreign exchange loss / (gain)		**711**	(169)	78
Depreciation	13	**48**	38	23
Amortization of intangible fixed assets	12	**10**	10	10
Movement in provisions	19	**12**	28	28
Research and development expenditure credit	7	**(3)**	(44)	(39)
Share-based payment	6	**1,379**	1,160	961
Adjusted loss from operations before changes in working capital		**(22,696)**	(19,323)	(11,172)
Decrease in prepayments and other receivables		**492**	1,106	(2,188)
Increase in deferred income		**30,527**	—	—
Increase / (decrease) in trade and other payables		**813**	(366)	1,693
Cash generated from / (used by) operations		**9,136**	(18,583)	(11,667)
Taxation received		**3,005**	1,401	658
Net cash generated from / (used by) operating activities		**12,141**	(17,182)	(11,009)
Investing activities				
Purchase of property, plant and equipment		**(81)**	(66)	(35)
Purchase of intangible assets		**(7)**	—	—
Interest received		**8**	30	51
Net cash used in investing activities		**(80)**	(36)	16
Financing activities				
Proceeds from issue of share capital		**—**	26,101	22,000
Transaction costs on share capital issued		**—**	(4,187)	(1,482)
Proceeds from exercise of warrants		**107**	—	—
Proceeds from exercise of share options		**283**	222	26
Cash received from funding arrangements accounted for as financial liabilities		**23**	—	423
Cash paid in respect of financial liabilites from funding arrangements		**—**	—	(739)
Net cash generated from financing activities		**413**	22,136	20,228
Increase in cash and cash equivalents		**12,474**	4,918	9,235
Effect of exchange rates in cash and cash equivalents		**(716)**	121	—
Cash and cash equivalents at beginning of the year		**16,304**	11,265	2,030
Cash and cash equivalents at end of the year		**28,062**	16,304	11,265

* See Note 1 ‘Change in accounting policy’

The accompanying notes form an integral part of these Consolidated Financial Statements.

Consolidated Statement of Changes in Equity
Year ended January 31, 2017, 2016 and 2015

Year ended January 31, 2017

Group	Share capital £000s	Share premium account £000s	Share-based payment reserve £000s	Merger reserve £000s	Special reserve £000s	Currency translation reserve £000s	Accumulated losses reserve £000s	Total Equity £000s
At February 1, 2016 (Adjusted*)	613	46,035	3,757	(1,943)	19,993	21	(52,396)	16,080
Loss for the year	—	—	—	—	—	—	(21,371)	(21,371)
Currency translation adjustment	—	—	—	—	—	29	—	29
Total comprehensive loss for the year	—	—	—	—	—	29	(21,371)	(21,342)
New share capital issued from exercise of warrants	2	105	—	—	—	—	—	107
Share options exercised	3	280	—	—	—	—	—	283
Share-based payment	—	—	1,379	—	—	—	—	1,379
At January 31, 2017	618	46,420	5,136	(1,943)	19,993	50	(73,767)	(3,493)

Year ended January 31, 2016 (Adjusted*)

Group	Share capital £000s	Share premium account £000s	Share-based payment reserve £000s	Merger reserve £000s	Special reserve £000s	Currency translation reserve £000s	Accumulated losses reserve £000s	Total Equity £000s
At February 1, 2015	411	24,101	2,597	(1,943)	19,993	62	(32,259)	12,962
Loss for the year	—	—	—	—	—	—	(20,137)	(20,137)
Currency translation adjustment	—	—	—	—	—	(41)	—	(41)
Total comprehensive loss for the year	—	—	—	—	—	(41)	(20,137)	(20,178)
New share capital issued	198	25,903	—	—	—	—	—	26,101
Transaction costs on share capital issued	—	(4,187)	—	—	—	—	—	(4,187)
Share options exercised	4	218	—	—	—	—	—	222
Share-based payment	—	—	1,160	—	—	—	—	1,160
At January 31, 2016	613	46,035	3,757	(1,943)	19,993	21	(52,396)	16,080

Year ended January 31, 2015 (Adjusted*)

Group	Share capital £000s	Share premium account £000s	Share-based payment reserve £000s	Merger reserve £000s	Special reserve £000s	Currency translation reserve £000s	Accumulated losses reserve £000s	Total Equity £000s
At February 1, 2014	10,075	40,177	1,636	(1,943)	—	—	(47,166)	2,779
Loss for the year	—	—	—	—	—	—	(11,384)	(11,384)
Currency translation adjustment	—	—	—	—	—	62	—	62
Total comprehensive loss for the year	—	—	—	—	—	62	(11,384)	(11,322)
New share capital issued	3,384	18,616	—	—	—	—	—	22,000
Transaction costs on share capital issued	—	(1,482)	—	—	—	—	—	(1,482)
Cancellation of Deferred Shares	(13,048)	—	—	—	13,048	—	—	—
Reduction of share premium account	—	(33,236)	—	—	33,236	—	—	—
Elimination of losses	—	—	—	—	(26,291)	—	26,291	—
Share options exercised	—	26	—	—	—	—	—	26
Share-based payment	—	—	961	—	—	—	—	961
At January 31, 2015	411	24,101	2,597	(1,943)	19,993	62	(32,259)	12,962

* See Note 1—'Change in accounting policy'

The accompanying notes form an integral part of these Consolidated Financial Statements.

Share capital and premium

When shares are issued, the nominal value of the shares is credited to the share capital reserve. Any premium paid above the nominal value is credited to the share premium reserve. Ordinary Shares of Summit Therapeutics plc have a nominal value of 1 pence per share.

Share-based payment reserve

The share-based payment reserve arises as the expense of issuing share-based payments is recognized over time (share option grants). The reserve will fall as share options vest and are exercised, and the impact of the subsequent dilution of earnings crystallizes, but the reserve may equally rise or might see any reduction offset, as new potentially dilutive share options are issued.

Merger reserve

The merger reserve brought forward relates to the difference between the nominal value of Summit (Oxford) Limited arising from the Group reconstruction in 2004, accounted for using the merger method of accounting under UK GAAP, and the amount arising through application of S131 CA85, which is equal to the difference between nominal and fair value of shares issued in business combinations using the acquisition method of accounting.

Accumulated losses reserve

The accumulated losses reserve records the accumulated profits and losses, less any subsequent elimination of losses, of the Group since inception of the business. Where businesses or companies are acquired, only the profits or losses arising from the date of acquisition are included.

Special reserve

The special reserve was created during the consolidation and subdivision of the Company's share capital as part of a capital reorganization completed in September 2014. It represents the net balance of the cancellation of the Deferred Shares, the reduction of the share premium account and elimination of current losses from the accumulated deficit.

Currency translation reserve

The currency translation reserve records the foreign exchange difference that arises on the translation of the US subsidiary, Summit Therapeutics Inc.

Notes to the Financial Statements

1. Basis of accounting

The principal accounting policies adopted by Summit Therapeutics plc and its subsidiaries in the preparation of these financial statements are set out below. These policies have been consistently applied to all the years presented, unless otherwise stated.

Basis of preparation

The consolidated financial statements have been prepared in accordance with International Financial Reporting Standards ('IFRS') as issued by the IASB and the Companies Act 2006 applicable to companies reporting under IFRS. The Consolidated Financial Statements have been prepared on a going concern basis and under the historical cost convention. These consolidated financial statements were authorized by the Board of Directors on March 29, 2017.

Going concern

The financial information in these financial statements has been prepared on a going concern basis which assumes that the Group will continue in operational existence for the foreseeable future.

The Group expects it will need to raise additional funding in the future in order to support research and development efforts, potential commercialization-related activities if any of its product candidates receive marketing approval, as well as to support activities associated with operating as a public company in both the United States and the United Kingdom. Management expects to finance its cash needs through a combination of some, or all, of the following: equity offerings, collaborations, strategic alliances, grants and clinical trial support from government entities, philanthropic, non-government and not for profit organizations and patient advocacy groups, debt financings, and marketing, distribution or licensing arrangements.

After review of the future operating costs of the business in conjunction with the cash held at January 31, 2017, management is confident about the Group's ability to continue as a going concern.

Use of estimates

The preparation of the financial statements, in conformity with IFRS, requires the use of estimates and assumptions that affect the reported amounts of assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Although these estimates are based on management's best knowledge of the amount, event or actions, actual results may ultimately differ from those estimates. The areas involving higher degree of judgement or complexity, or areas where assumptions and estimates are significant to the consolidated financial statements are disclosed in Note 2 'Critical accounting judgements and key sources of estimation uncertainty'.

Basis of consolidation

The Consolidated Financial Statements incorporate the financial statements of the Group and entities controlled by the Group made up to the reporting date. Control is achieved where the Company has the power to govern the financial and operating policies of an investee entity so as to obtain benefits from its activities.

The results of subsidiary undertakings acquired or disposed of in the year are included in the Consolidated Statement of Comprehensive Income from the effective date of acquisition or up to the effective date of disposal, as appropriate. Where necessary, adjustments are made to the financial statements of subsidiaries to bring the accounting policies used into line with those used by the Group.

1. Basis of accounting (continued)

All intra-group transactions, balances, income and expenses are eliminated on consolidation.

Change in accounting policy

Following an IFRS Interpretations Committee agenda decision in May 2016 on the application of IAS 20 'Accounting for Government Grants and Disclosure of Government Assistance,' the Company has changed its accounting policy regarding charitable funding arrangements from the Wellcome Trust and the US not for profit organizations, the Muscular Dystrophy Association ('MDA') and Duchenne Partners Fund ('DPF'), which has resulted in an adjustment to the comparative financial statements.

In exchange for the funding provided, these arrangements require the company to pay royalties on potential future revenues generated from these projects and also give the counterparties certain rights over the intellectual property if the compound is not exploited. The IFRS Interpretations Committee decision has clarified that such arrangements result in a financial liability. The estimate of each financial liability is initially recognized at fair value using a discounted cash flow model with the difference between the fair value of the liability and the cash received considered to represent a charitable grant.

When determining the fair value on initial recognition, the significant assumptions in the models include the estimation of the timing and the probability of successful development leading to commercialization of the project related results and related estimates of future cash flows. Estimated future cash flows include expected sources of revenue (including commercial sales and upfront payments, milestone payments and royalties from potential licensing arrangements) and are calculated using estimated geographical market share and associated pricing.

The financial liabilities are subsequently measured at amortized cost using a discounted cash flow model which calculates the risk adjusted net present values of estimated potential future cash flows for the respective projects related to the Wellcome Trust and MDA and DPF agreements. The financial liabilities are re-measured when there is a specific significant event that provides evidence of a significant change in the probability of successful development such as the completion of a phase of research or changes in use or market for a product. The models will be updated for changes in the clinical probability of success and other associated assumptions with the discount factor to remain unchanged within the model.

Re-measurements of the financial liabilities are recognized in the income statement as finance costs. Grant income is recognized as other operating income in accordance with IAS 20, 'Accounting for Government Grants and Disclosure of Government Assistance,' at the same time as the underlying expenditure is incurred, provided that there is reasonable assurance that the Group will comply with the conditions.

Amounts received from, and subsequent payments to, the corresponding counterparty in the funding agreement which relate to the financial liability will be presented within the financing activities section in the Consolidated Statement of Cash Flows.

This change in accounting policy has been reflected retrospectively in these financial statements.

The impact of this change in accounting policy on the consolidated financial statements is a reduction in other income historically recognized, a change in the level of accrued income accounted for as grant income and the

1. Basis of accounting (continued)

recognition of a financial liability and finance costs associated with the unwinding of the discount and re-measurement of the liability.

Impact on Consolidated Interim Statement of Comprehensive Income	Original Year ended January 31, 2015 £000	Adjusted Year ended January 31, 2015 £000	Impact £000
Other operating income	2,148	1,888	(260)
General and administration	(4,442)	(3,704)	738
Finance costs	—	(499)	(499)
	(2,294)	(2,315)	(21)

Impact on Consolidated Interim Statement of Comprehensive Income	Original Year ended January 31, 2016 £000	Adjusted Year ended January 31, 2016 £000	Impact £000
Other operating income	1,451	1,281	(170)
Finance costs	—	(2,879)	(2,879)
	1,451	(1,598)	(3,049)

Impact on Consolidated Statement of Financial Position	Original February 1, 2015 £000	Adjusted February 1, 2015 £000	Impact £000
Trade and other payables	(3,721)	(3,570)	151
Financial liabilities on funding arrangements	—	(2,155)	(2,155)
Accumulated losses reserve	(30,255)	(32,259)	(2,004)

Impact on Consolidated Statement of Financial Position	Original January 31, 2016 £000	Adjusted January 31, 2016 £000	Impact £000
Prepayments and other receivables	1,538	1,519	(19)
Financial liabilities on funding arrangements	—	(5,034)	(5,034)
Accumulated losses reserve	(47,343)	(52,396)	(5,053)

Impact on Consolidated Statement of Cash Flows	Original Year ended January 31, 2015 £000	Adjusted Year ended January 31, 2015 £000	Impact £000
Loss before income tax	(12,660)	(12,681)	(21)
Adjusted for:			
Finance costs	—	499	499
Increase in prepayments and other receivables	(2,200)	(2,188)	12
Increase in trade and other payables	1,867	1,693	(174)
Net cash used in operating activities	(11,325)	(11,009)	316
Cash received from funding arrangements accounted for as financial liabilities	—	423	423
Cash paid in respect of financial liabilities from funding arrangements	—	(739)	(739)
Net cash generated from financing activities	20,544	20,228	(316)

1. Basis of accounting (continued)

Impact on Consolidated Statement of Cash Flows	Original Year ended January 31, 2016 £000	Adjusted Year ended January 31, 2016 £000	Impact £000
Loss before income tax	(20,146)	(23,195)	(3,049)
Adjusted for:			
Finance costs	—	2,879	2,879
Decrease in trade and other payables	(536)	(366)	170
Impact on net cash used in operating activities	(20,682)	(20,682)	—

Revenue recognition

Revenue is measured at the fair value of the consideration received or receivable and represents amounts receivable for goods and services provided in the normal course of business net of value added tax and other sales-related taxes. The Group recognizes revenue when the amount can be reliably measured; when it is probable that future economic benefits will flow to the Group; and when specific criteria have been met for each of the Group's activities.

Collaboration revenues consist of revenues generated from collaborative research and development arrangements. Such agreements may consist of multiple elements and provide for varying consideration terms, such as upfront, development, regulatory and sales milestones, and sales royalties and similar payments. Where such arrangements can be divided into separate units of accounting (each unit constituting a separate earnings process), the arrangement consideration is allocated to the different units based on their relative fair values and recognized over the respective performance period.

Revenues from non-refundable, upfront payments are assessed as to whether they relate to the provision of a license or development services. Upfront payments classified as the provision of a license are recognized in full immediately while revenue related to further development services are initially reported as deferred income on the Consolidated Statement of Financial Position and are recognized as revenue over the development period.

Development and regulatory approval milestone payments are recognized as revenue based on the percentage of completion method on the assumption that all stages will be completed successfully, but with cumulative revenue recognized limited to non-refundable amounts already received or reasonably certain to be received.

Royalty revenue is recognized on an accrual basis in accordance with the substance of the relevant agreement, provided that it is probable that the economic benefits will flow to the Group and the amount of revenue can be measured reliably.

Sales related milestone payments are recognized in full in the period in which the relevant milestone is achieved.

Business Combinations

The cost of an acquisition is measured as the fair value of the assets exchanged, equity instruments issued and liabilities incurred or assumed at the date of exchange. Identifiable assets acquired together with liabilities and contingent liabilities assumed in a business combination are measured initially at their fair values at the acquisition date. The excess of the cost of acquisition over the fair value of the identifiable net assets is recorded as goodwill. Goodwill is not amortized but is reviewed for impairment at least annually and more frequently whenever there is an indication of impairment.

1. Basis of accounting (continued)

Intangible Assets

In-process research and development that is separately acquired as part of a company acquisition or in-licensing agreement is capitalized even if they have not yet demonstrated technical feasibility, which is usually signified by regulatory approval. The intangible asset relating to intellectual property rights for the utrophin program capitalized as part of the acquisition of MuOx Limited in November 2013 is considered to be not yet available for use. As such, it will not be subject to amortization and will be tested for impairment at least annually or whenever there is an indicator of impairment. Amortization will commence when either products underpinned by the intellectual property rights or the rights themselves become available for use.

Other intangible assets, comprising patents are amortized in equal installments over their useful estimated lives as follows:

All patents (once filed):	Over the period of the relevant patents (assumed to be 20 years)

Impairment of assets

At each year end date, the Group reviews the carrying amounts of its tangible and intangible assets to determine whether there is any indication that those assets have suffered an impairment loss.

For the purposes of assessing impairment, assets are grouped at the lowest levels for which there are separately identifiable cash flows (cash-generating units).

An impairment loss is recognized for the amount by which the asset's or cash-generating unit's carrying amount exceeds its recoverable amount. The recoverable amount is the higher of fair value, reflecting market conditions less costs to sell, and value in use based on an internal discounted cash flow evaluation. Impairment losses recognized for cash-generating units is charged *pro rata* to the other assets in the cash generating unit. All tangible and intangible assets are subsequently reassessed for indications that an impairment loss previously recognized may no longer exist. See Note 12 'Intangible assets' for details.

Property, plant and equipment

Property, plant and equipment are stated at cost less depreciation. Cost comprises the purchase price plus any incidental costs of acquisition and commissioning. Depreciation is calculated to write-off the cost, less residual value, in equal annual installments over their estimated useful lives as follows:

Leasehold improvements	Over the period of the remaining lease
Laboratory equipment	3-10 years
Office and IT equipment	3-5 years

The residual value, if not insignificant, is reassessed annually.

Provisions

Provisions are recognized when the Group has a present obligation (legal or constructive) as a result of a past event, where it is probable that an outflow of resources will be required to settle the obligation, and where a reliable estimate can be made of the amount of the obligation. If the effect of the time value of money is material, the expected future cash flows will be discounted using a pre-tax discount rate, adjusted for risk where it is inherent in a specific liability.

1. Basis of accounting (continued)

Other operating income

Other operating income includes income received and recognized from government agencies, philanthropic, non-government, not for profit organizations and patient advocacy groups which are accounted for in accordance with IAS 20, 'Accounting for Government Grants and Disclosure of Government Assistance'. Monies received through these means are held as deferred income in the Consolidated Statement of Financial Position and are released to the Consolidated Statement of Comprehensive Income as the underlying expenditure is incurred and to the extent the conditions of the grant are met.

Foreign currencies

Transactions in foreign currencies are recorded at the rate ruling at the date of the transaction. Monetary assets and liabilities denominated in foreign currencies are translated at the rate of exchange ruling at the year end date. All differences are taken to the Consolidated Statement of Comprehensive Income.

Assets and liabilities of subsidiaries that have a functional currency different from the presentation currency (Pound Sterling), are translated at the closing rate at the date of each statement of financial position presented. Income and expenses are translated at average exchange rates. Any resulting differences are recognized in other comprehensive income (loss) in the Consolidated Statement of Comprehensive Income.

Employee benefits

All employee benefit costs, notably holiday pay, bonuses and contributions to Company or personal defined contribution pension schemes are charged to the Consolidated Statement of Comprehensive Income on an accruals basis.

Leased assets

Costs in respect of operating leases are charged to the Consolidated Statement of Comprehensive Income on a straight line basis over the lease term. Assets relating to lease incentives are depreciated over the life of the lease and are included in property, plant and equipment as leasehold improvements.

Research and development

All ongoing research expenditure is currently expensed in the period in which it is incurred. Due to the regulatory environment inherent in the development of the Group's products, the criteria for development costs to be recognized as an asset, as set out in IAS 38 'Intangible Assets', are not met until a product has received regulatory approval, and it is probable that future economic benefit will flow to the Group. The Group currently has no qualifying expenditure.

Cash and cash equivalents

Cash and cash equivalents include cash in hand and deposits held on call with the bank.

Share-based payments

In accordance with IFRS 2 'Share-based Payment', share options are measured at fair value at their grant date. The fair value for the majority of the options is calculated using the Black-Scholes formula and charged to the Consolidated Statement of Comprehensive Income on a straight-line basis over the expected vesting period. For those options issued with vesting conditions other than remaining in employment (for example, those conditional upon the Group achieving certain predetermined financial criteria) either a Monte-Carlo model or a Hull White

1. Basis of accounting (continued)

trinomial lattice model have been used. At each year end date, the Group revises its estimate of the number of options that are expected to become exercisable. This estimate is not revised according to estimates of changes in market based conditions.

Current taxation

Income tax is recognized or provided at amounts expected to be recovered or paid using the tax rates and tax laws that have been enacted or substantively enacted at the year end date.

Current tax includes research and development tax credits which are calculated in accordance with the UK research and development tax credit regime applicable to small and medium sized companies. Research and development expenditure which is not eligible for reimbursement under the small and medium sized companies regime, such as expenditure incurred on projects for which we receive income, may be reimbursed under the UK Research and Development Expenditure Credit ('RDEC') scheme. Receipts under the RDEC scheme are presented within other operating income as they are similar in nature to grant income.

Deferred taxation

Deferred tax assets and liabilities are recognized where the carrying amount of an asset or liability in the Consolidated Statement of Financial Position differs from its tax base, except for differences arising on:

- the initial recognition of goodwill;
- the initial recognition of an asset or liability in a transaction which is not a business combination and at the time of the transaction affects neither accounting or taxable profit; and
- investments in subsidiaries and jointly controlled entities where the Group is able to control the timing of the reversal of the difference, and it is probable that the difference will not reverse in the foreseeable future.

Recognition of deferred tax assets is restricted to those instances where it is probable that taxable profit will be available against which the difference can be utilized.

The amount of the asset or liability is determined using tax rates that have been enacted or substantively enacted by the reporting date and are expected to apply when the deferred tax liabilities/(assets) are settled/(recovered).

Financial instruments

The Group holds financial assets and liabilities in the respective categories 'Loans and receivables' and 'Financial liabilities measured at amortized cost'. Loans and receivables are non-derivative financial assets with fixed or determinable payments that are not quoted in an active market. They arise when the Group provides money, goods or services directly to the debtor with no intention of trading the receivable. They are included in current assets, except for maturities greater than 12 months after the year end date, which are classified as non-current assets. Other liabilities consist of trade and other payables, being balances arising in the course of normal business with suppliers, contractors and other service providers, and borrowings, being loans and hire purchase funds advanced for the refit of leasehold premises and the purchase of laboratory equipment, fixtures and fittings. Loans and receivables, and other liabilities are initially recorded at fair value, and thereafter at amortized cost, if the timing difference is deemed to impact the fair value of the asset or liability.

The Group assesses at each year end date whether there is objective evidence that a financial asset or a group of financial assets is impaired.

The Group does not hold or trade in derivative financial instruments.

1. Basis of accounting (continued)

Warrants

Warrants issued by the Group are recognized and classified as equity when upon exercise, the Company would issue a fixed amount of its own equity instruments (Ordinary Shares) in exchange for a fixed amount of cash or another financial asset.

Consideration received, net of incremental costs directly attributable to the issue of such new warrants, is shown in equity. Such warrants are not re-measured at fair value in subsequent reporting periods.

2. Critical accounting judgements and key sources of estimation uncertainty

The preparation of the Consolidated Financial Statements requires the Group to make judgements, estimates and assumptions that affect the application of accounting policies and the reported amounts of assets and liabilities, income and expense. Actual results may differ from those estimates.

Critical Judgements in Applying the Group's Accounting Policies

The following are the critical judgements, apart from those involving estimations, that the Directors have made in the process of applying the Group's accounting policies and that have the most significant effect on the amounts recognized in the Consolidated Financial Statements.

Financial liabilities on funding arrangements

When entering into funding agreements with charitable and not for profit organizations, management is required to assess whether, based on the terms of the agreement, they can avoid a transfer of cash only by settling a non-financial obligation. An example of this would be the obligation to transfer the rights to the research to a funding provider. In the circumstances where the Group cannot avoid the obligation, all or part of the funding agreement should be accounted for as a financial liability rather than as a charitable grant. The financial liabilities are re-measured, and the Group is required to apply judgement, when there is a specific significant event that provides evidence of a significant change in the probability of successful development such as the completion of a phase of research or changes in use or market for a product. See Note 17 'Financial liabilities on funding arrangements'.

Revenue Recognition

The Group recognizes revenue from licensing fees, collaboration fees, development, regulatory and approval milestone fees, sales milestones and royalties. Agreements generally include a non-refundable up-front fee, milestone payments, the receipt of which is dependent upon the achievement of certain clinical, regulatory or commercial milestones, as well as royalties on product sales of licensed products, if and when such product sales occur. For these agreements, the Group is required to apply judgement in the allocation of total agreement consideration to the separately identifiable components on a reliable basis that reasonably reflects the selling prices that might be expected to be achieved in stand-alone transactions. The Group is required to make a judgement on those components which can be recognized immediately and those to which it applies the percentage of completion revenue recognition method. In relation to the license and collaboration with Sarepta, management has assessed that the development services to be indistinguishable from the license and as a result the upfront payment has been initially reported as deferred income on the Consolidated Statement of Financial Position and is being recognized as revenue over the development period. See Note 16 'Deferred income'.

Recognition of research expenditure

The Group recognizes expenditure incurred in carrying out its research and development activities in line with the management's best estimation of the stage of completion of each separately contracted study or activity. This

2. Critical accounting judgements and key sources of estimation uncertainty (continued)

includes the calculation of research and development accruals at each period to account for expenditure that has been incurred. This requires estimations of the full costs to complete each study or activity and also estimation of the current stage of completion. In all cases, the full cost of each study or activity is expensed by the time the final report or where applicable, product, has been received.

Key sources of estimation uncertainty

The key assumptions concerning the future, and other key sources of estimation uncertainty at the balance sheet date that may have a risk of causing a material adjustment to the carrying amounts of assets and liabilities within the next financial year, are noted below.

Financial liabilities on funding arrangements

In calculating the financial liability, both at inception and when it is subsequently re-measured, a number of assumptions need to be made by management which include significant estimates. Assumptions included in the model include the following: reported disease prevalence; expected market share based on management's estimates; drug reimbursement pricing in different territories, potential licensing terms which may be offered to the Group (for relevant products); expected patent life; the timing and probabilities of achieving clinical development milestones which are based on industry standards and adjusted for therapy area and; the appropriate discount rate to be used. See Note 17 'Financial liabilities on funding arrangements'.

Share-based payment

The Group measures share options at fair value at their grant date in accordance with IFRS 2, 'Share-based Payment.' The Group calculates the fair value of the share option using either the Black-Scholes model, or for options with performance conditions, a simulation model. The Group charges the fair value to the Consolidated Income Statement over the expected vesting period. See Note 22 'Share option scheme'.

3. Changes to accounting policies

During the year ended January 31, 2017 the following new standards, amendments to standards or interpretations became effective for the first time. The adoption of these interpretations, standards or amendment to standards were either not relevant for the Group or have not led to any significant impact on the Group's financial statements.

International Accounting Standards (IAS/IFRS)	Effective Date
Amendment to IFRS 5, Non-Current Assets Held for Sale and Discontinued Operations: Methods of disposal	January 1, 2016
Amendment to IFRS 7, Financial Instruments: Disclosures.	January 1, 2016
Amendment to IAS 19, Employee Benefits.	January 1, 2016
Amendment to IAS 34, Interim Financial Reporting	January 1, 2016
Amendment to IFRS 11, Accounting for Acquisitions of interests in Joint Operations	January 1, 2016
Amendment to IAS 16, Property, Plant and Equipment, and IAS 41, Agriculture, Regarding Bearer Plants.	January 1, 2016
Amendment to IAS 16, Property, Plant and Equipment and IAS 38, Intangible Assets, Clarification of Acceptable Methods of Depreciation and Amortisation	January 1, 2016
Amendment to IAS 27, Equity Method in Separate Financial Statements	January 1, 2016
Amendments to IFRS 10, Consolidated Financial Statements and IAS 28, Investments in Associates' on Investment Entities: Applying the Consolidation Exception	January 1, 2016
Amendment to IAS 1, 'Presentation of Financial Statements' on the Disclosure Initiative	January 1, 2016

3. Changes to accounting policies (continued)

At the date of signing these consolidated financial statements, the following standards, amendments and interpretations, which have not been applied in these financial statements, were in issue but not yet effective:

International Accounting Standards (IAS/IFRS)	Effective Date
IFRS 9, Financial Instruments (as revised in 2014)	January 1, 2018
IFRS 15, Revenue from Contracts with Customers	January 1, 2018
IFRS 16, Leases	January 1, 2019
Amendment to IFRS 2, Classification and Measurement of Share-based Payment Transactions	January 1, 2018
Amendment to IFRS 10 and IAS 28, Sale or Contribution of Assets between an Investor and its Associate or Joint Venture	To be determined
Amendment to IAS 7, Disclosure Initiative	January 1, 2017
Amendment to IAS 12, Recognition of Deferred Tax Assets for Unrealized Losses	January 1, 2017

IFRS 15 establishes comprehensive guidelines for determining when to recognize revenue and how much revenue to recognize. The core principle in that framework is that a company should recognize revenue to depict the transfer of control of promised goods or services to the customer in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The standard is effective for reporting periods beginning on or after January 1, 2018. The Group continues to assess the impact of IFRS 15 on the phasing of revenue recognition in connection with the Sarepta agreement as the guidance on the application for such arrangements develop. See Note 16 for additional detail on the Sarepta agreement.

IFRS 16 'Leases' will replace IAS 17 for accounting periods beginning on or after January 1, 2019. In so doing, it will eliminate the distinction between classification of leases as finance or operating leases for lessees. The adoption of IFRS 16 is not expected to have a significant impact on the Group's net results or net assets, although the full impact will be subject to further assessment following the conclusion of the ongoing consultations.

The Directors do not expect that the adoption of the remaining standards and interpretations in future periods will have a material impact on the financial statements of the Group.

4. Segmental reporting

The Summit Group comprises nine legal entities, of which three are trading. These included the eight subsidiary companies and the Group holding company, Summit Therapeutics plc. The Group operates in one reportable segment: Drug Development. The chief operating decision-maker has been identified as the Executive Management Team consisting of the Chief Executive Officer and the Chief Financial Officer. The Executive Management Team reviews the consolidated operating results regularly to make decisions about the financial and organizational resources and to assess overall performance.

The Drug Development segment covers Summit's research and development activities carried out by the Group, primarily comprising the DMD and the CDI programs.

The corporate and other activities of Summit Therapeutics plc, Summit (Oxford) Limited and Summit Therapeutics Inc which comprise the costs incurred in providing the facilities, finance, human resource and information technology services, are incurred by the main segment of the Group.

Substantially all of the Group's assets are held in the United Kingdom.

5. Revenue

	Year ended January 31, 2017 £000	Year ended January 31, 2016 £000
Analysis of revenue by category:		
Collaboration and license agreement	**2,304**	—
	2,304	—

Revenue recognized in the year relates to one single customer. See Note 16 'Deferred income' for further details.

	Year ended January 31, 2017 £000	Year ended January 31, 2016 £000
Analysis of revenue by geography:		
United States	**2,304**	—
	2,304	—

The analysis of revenue by geography has been identified on the basis of the customer's geographical location.

6. Directors and employees

The average monthly number of employees of the Group, including Executive Directors, during the year was:

	January 31, 2017	January 31, 2016
Technical, research and development	**23**	19
Corporate and administration	**21**	18
	44	37

The Parent Company had no employees in the current or previous financial years.

Their aggregate remuneration comprised:

	Year ended January 31, 2017 £000	Year ended January 31, 2016 £000
Wages and salaries	**5,932**	3,876
Social security costs	**434**	247
Other pension costs	**332**	90
Share-based payment	**1,379**	1,160
	8,077	5,373

6. Directors and employees (continued)

Key management of the Group are members of the Executive Management Team. The aggregate amounts of key management compensation are set out below:

	Year ended January 31, 2017 £000	Year ended January 31, 2016 £000
Short-term employee benefits		
Wages and salaries	**1,252**	934
Social security costs	**98**	58
	1,350	992
Post-employment benefits		
Amounts paid in lieu of employer pension contributions	**17**	17
Other pension costs	**11**	—
	28	17
Share-based payment	**327**	626
Total remuneration	**1,705**	1,635

7. Loss before income tax

	Year ended January 31, 2017 £000	Year ended January 31, 2016 Adjusted* £000
Other operating income		
Income recognized in respect of the Wellcome Trust	**13**	592
Grant income(1)	**56**	645
Research and development credit	**3**	44
	72	1,281
Research and development		
Employee benefit expense	**4,218**	2,848
Share-based payment expense	**374**	356
Program related costs	**13,605**	13,093
Amortization of intangible assets	**10**	10
Other research and development costs	**745**	549
	18,952	16,856
General and administration		
Employee benefit expense	**2,480**	1,365
Share-based payment expense	**1,005**	804
Foreign exchange loss / (gain)	**533**	(501)
Depreciation of property, plant and equipment	**48**	38
Operating lease rentals	**213**	131
Other general and administration costs	**3,998**	2,934
	8,277	4,771

(1) Grant income includes amounts received from Innovate UK.

* See Note 1 ‘Change in accounting policy’

8. Auditors' remuneration

Services provided by the Group's auditors

During the year the Group obtained the following services from the Group's auditors at the cost detailed below:

	Year ended January 31, 2017 £000	Year ended January 31, 2016 £000
Fees payable to the Company's auditors and its associates for the audit of the Parent Company and Consolidated Financial Statements	**110**	44
Fees payable to the Company's auditors and its associates for other services:		
- Audit of the Company's subsidiaries	**120**	71
- Audit-related assurance services	**3**	6
- Other assurance services(1)	**163**	158
- Tax advisory services	**15**	9
- Tax compliance services	**47**	11
Total fees payable	**458**	299

(1) For the year ended January 31, 2017, other assurance services includes assurance reporting on information included in the Company's F-3 registration statement that was originally filed with the US Securities and Exchange Commission on 12 May 2016.

9. Income tax

	Year ended January 31, 2017 £000	Year ended January 31, 2016 Adjusted £000
Analysis of credit in the period:		
Current tax: United Kingdom corporation tax at 20% (2016—20.17%)		
Current tax income	**4,245**	2,971
Adjustments in respect of prior years	**(9)**	87
Total current tax	**4,236**	3,058
Total deferred tax	**100**	—
Total tax	**4,336**	3,058

9. Income tax (continued)

The difference between the total tax shown above and the amount calculated by applying the standard rate of UK corporation tax to the loss before tax is as follows:

	Year ended January 31, 2017	Year ended January 31, 2016 Adjusted
	£000	£000
Loss before tax	**(25,707)**	(23,195)
Loss multiplied by the standard rate of corporation tax in the United Kingdom (Current tax) 20% (2016: 20.17%)	**(5,141)**	(4,678)
Change in unrecognized tax losses	**2,169**	2,691
Non-deductible expenses	**331**	184
Tax relief for qualifying research and development expenditure	**(1,699)**	(1,170)
Prior year adjustments	**9**	(87)
Share options exercised	**(84)**	(45)
Taxable losses at foreign rates	**179**	47
Change in rate of deferred tax	**(100)**	—
Total tax	**(4,336)**	(3,058)

There are no current tax liabilities as at January 31, 2017 (2016: Nil).

Tax credits relate to UK research and development tax credits claimed under Finance Act 2015.

The Finance (No 2) Act 2015, which provides for reductions in the main rate of corporation tax from 20% to 19% effective from April 1, 2017, and to 18% effective from April 1, 2020, was substantively enacted on October 26, 2015. Subsequently, the Finance Act 2016, which provides for a further reduction in the main rate of corporation tax to 17% effective from April 1, 2020, was substantively enacted on September 6, 2016. These rate reductions have been reflected in the calculation of deferred tax at the balance sheet date.

The closing deferred tax liability at January 31, 2017 has been calculated at 17% reflecting the tax rate at which the deferred tax liability is expected to be reversed in future periods. Unrecognized deferred tax has been calculated at 17% reflecting the latest enacted rate. In respect of unrecognized deferred tax on losses, the new loss restriction rules which are announced but not substantively enacted are expected to limit the amount of brought forward losses available to use against future taxable profits on a year by year basis to the extent that taxable profits exceed £5m in year. However, the losses will not lapse and therefore the full amount will be relieved over time.

Please see Note 20 ‘Deferred tax liability’ for information on the unrecognized tax losses carried forward.

10. Loss per share

The loss per share has been calculated using the loss for the year £21,371,000 (year ended January 31, 2016: loss of £20,137,000) and dividing this by the weighted average number of Ordinary Shares in issue during the year to January 31, 2017: 61,548,557 (year ended January 31, 2016: 59,102,292).

Since the Group has reported a net loss, diluted loss per share is equal to basic loss per share.

Potentially dilutive shares capable of vesting under the share options currently in issue totalled 7,383,401 as at January 31, 2017 (January 31, 2016: 7,006,306).

11. Goodwill

	MuOx Limited £000s	Total £000s
Cost		
At February 1, 2016	664	664
At January 31, 2017	664	664
Accumulated amortization		
At February 1, 2016	—	—
At January 31, 2017	—	—
Net book amount		
At February 1, 2016	664	664
At January 31, 2017	664	664

	MuOx Limited £000s	Total £000s
Cost		
At February 1, 2015	664	664
At January 31, 2016	664	664
Accumulated amortization		
At February 1, 2015	—	—
At January 31, 2016	—	—
Net book amount		
At February 1, 2015	664	664
At January 31, 2016	664	664

Goodwill represents the difference between the fair value of the identifiable assets acquired and liabilities assumed for MuOx Limited and the amount paid in consideration**.** Goodwill is attributable to synergies expected from the Group's collaboration with the University of Oxford and other founders of MuOx Limited.

In accordance with IAS 36 'Goodwill' has been reviewed for impairment and no provision is considered necessary. The impairment review is included as part of the intangible assets impairment review in Note 12 'Intangible assets' as they form part of the same cash-generating unit.

12. Intangible assets

	Iminosugar related programs acquired £000s	Utrophin program acquired £000s	Other patents and licenses £000s	Total £000s
Cost				
At February 1, 2016	1,380	3,321	197	4,898
Additions	—	—	7	7
At January 31, 2017	1,380	3,321	204	4,905
Accumulated amortization				
At February 1, 2016	(1,380)	—	(45)	(1,425)
Charge for the year	—	—	(10)	(10)
At January 31, 2017	(1,380)	—	(55)	(1,435)
Net book amount				
At February 1, 2016	—	3,321	152	3,473
At January 31, 2017	—	3,321	149	3,470

	Iminosugar related programs acquired £000s	Utrophin program acquired £000s	Other patents and licenses £000s	Total £000s
Cost				
At February 1, 2015	1,380	3,321	197	4,898
At January 31, 2016	1,380	3,321	197	4,898
Accumulated amortization				
At February 1, 2015	(1,380)	—	(35)	(1,415)
Charge for the year	—	—	(10)	(10)
At January 31, 2016	(1,380)	—	(45)	(1,425)
Net book amount				
At February 1, 2015	—	3,321	162	3,483
At January 31, 2016	—	3,321	152	3,473

Amortization of intangible assets is included in the line 'Research and development' shown on the face of the Consolidated Statement of Comprehensive Income.

In accordance with IAS 38, 'Intangible assets' have been reviewed for impairment.

The key assumptions used in the valuation model to determine the value in use are as follows:

- expected research and development costs based on management's past experience and knowledge;
- probabilities of achieving development milestones based on industry standards;
- reported disease prevalence;
- expected market share based on management's estimates;
- drug reimbursement, costs of goods and marketing estimates; and
- expected patent life.

12. Intangible assets (continued)

The valuation model covers a period significantly longer than five years which is based on expected patent life, once filed, due to the length of the development cycle for assets of this nature. A discount factor of 18% has been used over the forecast period.

Based on sensitivity analysis, no reasonably possible change in assumptions would cause the carrying value of this asset to exceed its recoverable amount.

13. Property, plant and equipment

Cost	Leasehold improvements £000s	Laboratory equipment £000s	Office and IT equipment £000s	Total £000s
At February 1, 2016	9	137	228	374
Additions	—	—	81	81
Disposals	—	(118)	(25)	(143)
At January 31, 2017	**9**	**19**	**284**	**312**
Accumulated depreciation				
At February 1, 2016	(7)	(135)	(149)	(291)
Charge for the year	(2)	—	(46)	(48)
Disposals	—	118	25	143
At January 31, 2017	**(9)**	**(17)**	**(170)**	**(196)**
Net book value				
At February 1, 2016	2	2	79	83
At January 31, 2017	**—**	**2**	**114**	**116**

Cost	Leasehold improvements £000s	Laboratory equipment £000s	Office and IT equipment £000s	Total £000s
At February 1, 2015	9	137	162	308
Additions	—	—	66	66
At January 31, 2016	**9**	**137**	**228**	**374**
Accumulated depreciation				
At February 1, 2015	(4)	(135)	(114)	(253)
Charge for the year	(3)	—	(35)	(38)
At January 31, 2016	**(7)**	**(135)**	**(149)**	**(291)**
Net book value				
At February 1, 2015	5	2	48	55
At January 31, 2016	**2**	**2**	**79**	**83**

14. Prepayments and other receivables

	January 31, 2017 £000	January 31, 2016 Adjusted* £000
Other receivables	**342**	312
Prepayments and accrued income	**685**	1,207
	1,027	1,519

15. Trade and other payables

	January 31, 2017 £000	January 31, 2016 £000
Trade payables	**906**	716
Other taxes and social security	**94**	79
Accruals	**2,884**	2,310
Other creditors	**100**	101
	3,984	3,206

16. Deferred income

	January 31, 2017 £000	January 31, 2016 £000
Due within one year	**6,912**	—
Due more than one year	**23,615**	—

On October 4, 2016, Summit announced its entry into an exclusive license and collaboration agreement (the 'Agreement') with Sarepta Therapeutics Inc. ('Sarepta'), pursuant to which Summit granted Sarepta the exclusive right to commercialize products in the Group's utrophin modulator pipeline in the European Union, Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States (the 'Licensed Territory'). Such products include the Group's lead product candidate, ezutromid, for the treatment of Duchenne muscular dystrophy and its pipeline of second generation and future generation small molecule utrophin modulators. The Group also granted Sarepta an option to expand the Licensed Territory to include specified countries in Central and South America ('the Latin America Option'). The Group retains commercialization rights in the rest of the world.

Under the terms of the Agreement, Summit received an upfront payment of $40.0 million (£32.8 million) from Sarepta. The terms of the contract have been assessed, and the Group believes the development services to be indistinguishable from the license and as a result the upfront payment has been initially reported as deferred income on the Consolidated Statement of Financial Position and is being recognized as revenue over the development period. In addition, the Group will be eligible for potential future ezutromid-related development, regulatory and sales milestone payments totalling up to $522 million. This includes $42 million in respect of specified development milestones (including a $22 million milestone upon the first dosing of the last patient in Summit's PhaseOut DMD trial, payable on or after April 1, 2017), $150 million in respect of specified regulatory milestones and $330 million from specified sales milestones. Summit is also eligible for escalating royalties ranging from a low to high teens percentage of net sales in the Licensed Territories. Summit is also eligible to receive development and regulatory milestones related to its pipeline of second generation and future generation utrophin modulator candidates.

As part of the Agreement with Sarepta, the Group has committed to a specified level of expenditure in the performance of its activities prior to the end of calendar year 2019 under a development plan agreed between the parties.

17. Financial liabilities on funding arrangements

The Group has entered into charitable funding arrangements with the Wellcome Trust and the US not for profit organizations, the Muscular Dystrophy Association ('MDA') and Duchenne Partners Fund ('DPF'). In exchange for the funding provided, these arrangements require the Company to pay royalties on potential future revenues generated from these projects and also give the counterparties certain rights over the intellectual property if the

17. Financial liabilities on funding arrangements (continued)

compound is not exploited. A recent IFRS Interpretations Committee agenda decision has clarified that such arrangements result in a financial liability. The estimate of each financial liability is initially recognized at fair value using a discounted cash flow model with the difference between the fair value of the liability and the cash received considered to represent a charitable grant.

When determining the fair value on initial recognition, the significant assumptions in the models include the estimation of the timing and the probability of successful development leading to commercialization of the project related results and related estimates of future cash flows. Estimated future cash flows include expected sources of revenue (including commercial sales and upfront payments, milestone payments and royalties from potential licensing arrangements) and are calculated using estimated geographical market share and associated pricing.

The financial liabilities are subsequently measured at amortized cost using discounted cash flow models which calculate the risk adjusted net present values of estimated potential future cash flows for the respective projects related to the Wellcome Trust and MDA and DPF agreements. The financial liabilities are re-measured when there is a specific significant event that provides evidence of a significant change in the probability of successful development such as the completion of a phase of research or changes in use or market for a product. The models will be updated for changes in the clinical probability of success and other associated assumptions with the discount factor to remain unchanged within the model. Discount factors have been calculated using appropriate measures and rates which could have been obtained in the period that the funding agreements were entered into and are in the range of 16% to 18%.

The value of the estimated financial liabilities for funding arrangements as of January 31, 2017 amounted to £5.9 million (January 31, 2016: £5.0 million). The increase in value of the estimated financial liabilities during the year ended January 31, 2016 amounted to £0.9 million (year ended January 31, 2016: £2.9 million) and was recognized as a finance expense. Since initial recognition, the estimated financial liabilities were re-measured following significant successful events in the DMD and CDI clinical programs. The financial liabilities were re-measured in the year ended January 31, 2016 following positive data in the DMD and CDI clinical programs which increased the probabilities of success.

	January 31, 2017 £000	January 31, 2016 Adjusted £000
At February 1	**5,034**	2,155
Unwinding of discount factor	**862**	268
Re-measurement of financial liabilities on funding arrangements	**—**	2,611
Total finance cost	**862**	2,879
Cash received from funding arrangements accounted for as financial liabilities	**23**	—
At January 31	**5,919**	5,034

17. Financial liabilities on funding arrangements (continued)

The table below describes the value of the liabilities as at January 31, 2017 of £5.9 million compared to what the total value would be following the presented variations to the underlying assumptions in both models:

	January 31, 2017 £000
Estimated financial liabilities on funding arrangements	5,919
1% lower discount rate	6,487
1% higher discount rate	5,414
10% lower revenue assumptions	5,336
10% higher revenue assumptions	6,455
10% lower probability of success	3,474
10% higher probability of success	8,328

Summary of milestone payments and royalty arrangements contained in the funding arrangements

Wellcome Trust

Under the terms of the revenue sharing agreement the Group would enter into with the Wellcome Trust to permit its exploitation of the exploitation intellectual property ('IP') or award products, the Wellcome Trust is entitled to a share of the cumulative net revenue that the Company or its affiliates receive from exploiting the exploitation IP or award products. The Wellcome Trust would be eligible to receive a tiered portion of the net revenue, ranging from a mid-single digit percentage up to a mid-twenties percentage. In addition, the Company would be obligated to pay the Wellcome Trust a milestone in a specified amount if cumulative net revenue exceeds a specified amount.

US Not for Profit Organisations

Muscular Dystrophy Association

The Group has agreed to pay the Muscular Dystrophy Association ('MDA') a specified lump sum amount, less the previously paid MDA cash infusion milestone payment, following the regulatory approval of any project product for use in the United States or European Union in the treatment of DMD or Becker Muscular Dystrophy ('BMD') and an additional specified sum upon achievement of a commercial milestone. The Group would be obligated to pay MDA a low single digit percentage royalty of worldwide net sales by the Group, its affiliates or licensees of any project product.

Duchenne Partners Fund Inc.

The Group has agreed to pay Duchenne Partners Fund Inc., ('DPF') a specified lump sum amount, less the previously paid DPF cash infusion milestone payment, following the regulatory approval of any project product for use in the United States or European Union in the treatment of DMD or BMD and an additional specified sum upon achievement of a commercial milestone. The Group would be obligated to pay DPF a low single digit percentage royalty of worldwide net sales by the Group, its affiliates or licensees of any project product.

The total amount payable with respect to regulatory milestones under the two agreements with the US not for profit organizations would be $2.5 million if the Group meets all regulatory milestones.

18. Financial instruments

	Note	January 31, 2017 £000	January 31, 2016 £000
Loans and receivables			
Other receivables(1)	**14**	**342**	312
Cash and cash equivalents		**28,062**	16,304
		28,404	16,616
Financial liabilities measured at amortized cost			
Trade and other payables	**15**	**3,984**	3,206
Financial liabilities on funding arrangements	**17**	**5,919**	5,034
		9,903	8,240

(1) Prepayments have been excluded as they are not considered to be a financial instrument.

The Group's activities expose it to a variety of financial risks: foreign currency risk; interest rate risk; credit risk; and liquidity risk.

The Group's principal financial instrument comprises cash and cash equivalents, and this is used to finance the Group's operations. Other financial instruments include other receivables and trade and other payables that arise directly from its operations. The category of other receivables all mature within one year.

The Group has compared fair value to book value for each class of financial asset and liability: no difference was identified other than in respect of financial liabilities on funding arrangements. The Group has a policy, which has been consistently followed, of not trading in financial instruments.

The Group consider the financial liabilities on funding arrangements to be a level 3 financial instrument and the fair value at the balance sheet date was calculated to be £8.3 million. The key inputs to the model are described more fully within Note 2 'Critical accounting judgements and key sources of estimation uncertainty'.

Foreign currency risk

Foreign currency risk refers to the risk that the value of a financial commitment or recognized asset or liability will fluctuate due to changes in foreign currency rates. The Group's net income and financial position, as expressed in Pounds Sterling, are exposed to movements in foreign exchange rates against the US Dollar and the Euro. The main trading currencies of the Group are Pounds Sterling, the US Dollar, and the Euro. The Group is exposed to foreign currency risk as a result of trading transactions, including the receipt of potential payments related to the Group's agreement with Sarepta, capital raises in the US and the translation of foreign bank accounts.

The exposure to foreign exchange is monitored by the Group's finance function. Exposures are generally managed through natural hedging *via* the currency denomination of cash balances and any realized impact currently is not material to the Group.

	January 31, 2017 £000	January 31, 2016 £000
Cash at bank and in hand		
Pounds Sterling	**8,969**	12,430
US Dollar	**19,093**	3,874
	28,062	16,304

18. Financial instruments (continued)

Interest rate risk

One of the risks arising from the Group's financial instruments is interest rate risk. The Group holds no derivative instruments to manage interest rate risk; instead the Group placed deposits surplus to short-term working capital requirements with a variety of reputable UK- and US-based banks and building societies. There were no amounts on short term deposits at the year end. These balances are placed at fixed rates of deposit with maturities between one month and three months.

The Group's cash and short-term deposits were as follows:

	January 31, 2017 £000	January 31, 2016 £000
On current account	**28,062**	16,304
	28,062	16,304

The interest rates for dated deposits were dependent on the rates offered by the Group's borrowers. The interest rate for short-term deposits is variable dependent on the rates offered by the Group's banks. During the year to January 31, 2017, the banking facilities returned an average rate after fees of 0.04% (2016: 0.22%).

The Group's exposure to interest rate risk is illustrated with regard to the opening and closing cash balances and the difference that an increase or decrease of 1% in interest rates would have made based on the average cash balance of £22,183,000 (2016: £13,785,000) in the year:

Year ended January 31, 2017	**-1%**	**Actual**	**+1%**
Interest rate	**—**	**0.04**	**1.04**
Interest received (£000)	**—**	**8**	**230**

Year ended January 31, 2016	**-1%**	**Actual**	**+1%**
Interest rate	**—**	**0.22**	**1.22**
Interest received (£000)	**—**	**30**	**168**

Credit risk

The credit risk with respect to customers is limited and the Group had no trade receivables outstanding at January 31, 2017.

Financial instruments that potentially expose the Group to concentrations of credit risk consist primarily of short-term cash investments and trade accounts receivable. Excess cash is invested in short-term money market instruments, including bank term deposits, money market and liquidity funds, and other debt securities provided by a variety of financial institutions with strong credit ratings; these investments typically bore minimal credit risk in the year.

Cash balances maintained during the year have been principally held with reputable UK- and-US based banks and building societies. We do not believe that this constituted a major credit risk.

As of January 31, 2017 and January 31, 2016, the majority of cash and cash equivalents were placed with HSBC Bank plc.

18. Financial instruments (continued)

Liquidity risk

Prudent liquidity risk management implies maintaining sufficient cash and the availability of funding through an adequate amount of committed credit facilities.

The Group ordinarily finances its activities through cash generated from operating activities and private and public offerings of equity securities. The Group anticipates that its operating cash flow together with available cash and cash equivalents will be sufficient to meet its anticipated needs. See Note 1 'Going concern'.

All of the financial liability categories at each balance sheet date, excluding the financial liabilities on funding arrangements, have maturity dates of less than twelve months from the balance sheet date. Provisions are amounts contingent upon events taking place and the recognition of deferred taxation is dependent upon future profits arising.

Capital management

The primary aim of the Group's capital management, defined as its share capital, is to safeguard the Group's ability to continue as a going concern, to support its programs and maximize shareholder value.

The Group monitors its capital structure and makes adjustments, as and when it is deemed necessary and appropriate to do so, using such methods as the issuing of new shares. The capital structure of the Group has come entirely from equity issues.

19. Provisions for other liabilities and charges and contingent liabilities

Dilapidations	January 31, 2017 £000	January 31, 2016 £000
At February 1	**73**	45
Additions	**12**	28
At January 31	**85**	73

Management has made a provision in respect of the dilapidation costs associated with the reinstatement obligations on their current lease based on best estimates. It is management's intention to utilize the provision at the end of the lease term.

In addition to those items provided for above, the Group also has the following contingencies:

MuOx Limited

Under the option agreement that the Group and Oxford University Innovation Limited, formerly known as Isis Innovation Limited, ('OUI') entered into in November 2013, and as amended in November 2015, OUI granted to the Group an exclusive option to license the intellectual property ('IP') arising from the research carried out under the sponsored research agreement within specified periods. If the Group exercises its option to obtain a license under arising IP, the Group would be obliged to pay OUI up to a specified sum in option exercise fees.

For any IP arising from the research carried out under the sponsored research agreement and for which the Group has exercised the option and that comprises new chemical entities or compounds, the Group would obtain an exclusive, sub licensable license. The Group is obligated to pay milestone payments of up to £75,000 upon the achievement of specified development milestones, whether such milestones occur prior to or after the Group's exercise of the option to obtain an exclusive sub licensable license. Following exercise of such an option the

19. Provisions for other liabilities and charges and contingent liabilities (continued)

Group would also be obligated to pay milestone payments upon the achievement of specified regulatory milestones with respect to each optioned compound. The specified regulatory milestone payment is due each time the specified regulatory milestone is achieved with respect to an optioned compound and, if each optioned compound achieved each regulatory milestone, we would be obligated to pay OUI a total of £3.7 million in regulatory milestone payments for each optioned compound.

The Group would also be obligated to pay OUI a low single digit royalty of net sales by the Group, its affiliates or sub licensees of any product containing an optioned compound.

The School of Pharmacy, University of London

The Group has agreed to pay The School of Pharmacy, University of London, a low single-digit share of all revenue, pre and post commercialization, received by the Group in respect of ridinilazole up to a maximum of £1.0 million in consideration of their role in the development of the initial compound series from which ridinilazole was later identified.

20. Deferred tax liability

A deferred tax liability was recognized upon acquisition of MuOx Limited which took place in the year ended January 31, 2014.

	January 31, 2017 £000	January 31, 2016 £000
Amounts falling due after more than one year		
At February 1	**664**	664
Credited to the income statement	**(99)**	
At January 31	**565**	664

There is an unrecognized deferred tax asset in relation to the trading losses carried forward of £10,882,000 (2016: £9,579,000), £14,000 in relation to provisions (2016: £13,000) and £229,855 (2016: £184,000) in relation to future exercisable shares. There is a deferred tax liability of £3,000 (2016: £10,000) in respect of accelerated capital allowances, this has been offset against the deferred tax asset on in relation to trading losses carried forward.

The unrecognized deferred tax asset would be recovered against future company taxable profits. In the opinion of the Directors, there is insufficient evidence that the asset will be recovered, as such the deferred tax asset has not been recognized in the financial statements.

21. Share capital

	January 31, 2017 £000	January 31, 2016 £000
Allotted, called up and fully paid		
61,841,566 (2016: 61,290,740) Ordinary shares of 1p each	**618**	613
	618	613

On April 14, 2016 the number of Ordinary Shares increased to 61,467,785 following the exercise of warrants over 177,045 Ordinary Shares at an exercise price of 60 pence per share. The issue of shares raised net proceeds of £107,000.

21. Share capital (continued)

During the year to January 31, 2017, the following exercise of share options took place:

Date	Number of options exercised
June 28, 2016	16,667
October 6, 2016	238,804
October 7, 2016	77,500
October 14, 2016	3,560
October 24, 2016	11,000
January 19, 2017	26,250
	373,781

The total net proceeds from exercised share options during the year was £0.28 million.

Following the exercise of the above share options, the number of Ordinary Shares in issue was 61,841,566.

Post year end, on February 22, 2017, the number of Ordinary Shares increased to 61,891,566 following the exercise of warrants by Oxford University Innovation Limited, formerly known as Isis Innovation Limited, over 50,000 Ordinary Shares at an exercise price of 20 pence per share. The issue raised net proceeds of £10,000.

22. Share option scheme

At January 31, 2017, the outstanding share options, which include the share options granted to Directors, are shown below:

Date of grant	Exercise price (£)	Number of shares	Date from which exercisable	Expiry date
Approved EMI scheme				
November 21, 2007	2.28	4,800	November 21, 2008	November 21, 2017
April 07, 2011	0.65	5,873	April 08, 2014	April 07, 2021
May 10, 2012	0.60	150,046	May 10, 2014	May 10, 2022
December 24, 2012	0.85	54,000	December 24, 2015	December 24, 2022
January 31, 2013	0.20	72,973	July 31, 2013	January 31, 2023
July 15, 2014	1.26	347,121	July 15, 2016	July 15, 2024
January 21, 2015	1.23	25,000	January 21, 2017	January 21, 2025
June 23, 2016	1.05	660,952	June 23, 2017	June 23, 2026
		1,320,765		

22. Share option scheme (continued)

Date of grant	Exercise price (£)	Number of shares	Date from which exercisable	Expiry date
Unapproved scheme				
November 21, 2007	2.28	19,167	November 21, 2008	November 21, 2017
April 07, 2011	0.65	13,981	April 08, 2014	April 08, 2021
May 10, 2012	0.60	657,500	May 10, 2012	May 10, 2022
December 18, 2013	0.20	76,364	June 19, 2013	June 19, 2023
June 23, 2014	0.20	16,667	June 23, 2015	June 23, 2024
June 23, 2014	1.48	525,000	June 23, 2015*	June 23, 2024
July 15, 2014	1.26	975,000	July 15, 2016	July 15, 2024
July 15, 2014	0.80	100,000	May 30, 2015	May 30, 2023
December 23, 2014	1.37	25,000	December 23, 2016	December 23, 2024
January 21, 2015	1.23	75,000	January 21, 2017	January 21, 2025
June 16, 2015	1.43	2,402,333	June 16, 2017	June 16, 2025
September 04, 2015	1.49	120,000	September 04, 2017	September 04, 2025
October 15, 2015	1.31	50,000	October 15, 2017	October 15, 2025
June 23, 2016	0.01	110,576	July 21, 2016	June 23, 2026
June 23, 2016	1.05	250,000	June 23, 2019	June 23, 2026
June 23, 2016	1.05	646,048	June 23, 2017	June 23, 2026
		6,062,636		
		7,383,401		

* Subject to the achievement of performance conditions, these options will vest and become exercisable on completion of Phase 2 proof of concept clinical trials in both the DMD and CDI programs or the third anniversary of grant, whichever is sooner.

The Group has no legal or constructive obligation to repurchase or settle the options in cash.

The movement in the number of share options is set out below:

	Weighted average exercise price £	**Year ended January 31, 2017**	Weighted average exercise price £	Year ended January 31, 2016
Outstanding at February 1,	**1.29**	**7,006,306**	1.18	5,250,838
Granted during the year	**0.98**	**1,667,576**	1.43	2,592,333
Lapsed/surrendered during the year	**1.90**	**(916,700)**	1.31	(501,322)
Exercised during the year	**0.76**	**(373,781)**	0.66	(335,543)
Number of outstanding options at January 31,	**1.17**	**7,383,401**	1.29	7,006,306

As at January 31, 2017, 1,972,654 share options were capable of being exercised with a weighted average exercise price per option of 84 pence (2016: 1,987,296 with a weighted average exercise price per option of 98 pence). The options outstanding at January 31, 2017 had a weighted average exercise price per option of 117 pence (2016: 129 pence), and a weighted average remaining contractual life of 8.9 years (2016: 8.2 years).

22. Share option scheme (continued)

The fair value per award granted and the assumptions used in the calculations are as follows:

Date of grant	Type of award	Number of shares	Exercise price (£)	Share price at grant date (£)	Fair value per option (£)	Award life (years)	Risk free rate
November 21, 2007	EMI	4,800	2.28	2.28	0.84	3.00	4.60%
November 21, 2007	Unapproved	19,167	2.28	2.28	0.84	3.00	4.60%
April 07, 2011	EMI	5,873	0.65	0.65	0.47	5.00	2.70%
April 07, 2011	Unapproved	13,981	0.65	0.65	0.47	5.00	2.70%
May 10, 2012	EMI	150,046	0.60	0.52	0.24	5.00	1.00%
May 10, 2012	Unapproved	657,500	0.60	0.52	0.20	5.00	1.00%
December 24, 2012	EMI	54,000	0.85	0.85	0.59	5.00	0.90%
January 31, 2013	EMI	72,973	0.20	0.94	0.74	5.00	1.00%
December 18, 2013	Unapproved	76,364	0.20	1.85	1.65	5.00	1.00%
June 23, 2014	Unapproved	16,667	0.20	1.50	0.92	3.00	1.30%
June 23, 2014	Unapproved	525,000	1.47	1.50	0.92	3.80	1.30%
July 15, 2014	EMI	347,121	1.26	1.26	0.65	3.00	1.30%
July 15, 2014	Unapproved	975,000	1.26	1.26	0.65	3.00	1.30%
July 15, 2014	Unapproved	100,000	0.80	0.81	0.65	1.90	0.50%
December 23, 2014	Unapproved	25,000	1.37	1.37	0.70	3.00	0.80%
January 21, 2015	EMI	25,000	1.23	1.22	0.64	3.00	0.60%
January 21, 2015	Unapproved	75,000	1.23	1.22	0.64	3.00	0.60%
June 15, 2015	Unapproved	2,402,333	1.43	1.44	0.65	3.00	0.91%
September 04, 2015	Unapproved	120,000	1.49	1.48	0.68	3.00	0.88%
October 15, 2015	Unapproved	50,000	1.31	1.36	0.57	3.00	0.70%
23 June 2016	EMI	660,952	1.05	1.05	0.25	3.00	0.30%
23 June 2016	Unapproved	110,576	0.01	1.05	1.04	0.50	0.30%
23 June 2016	Unapproved	250,000	1.05	1.05	0.24	3.00	0.30%
23 June 2016	Unapproved	646,048	1.05	1.05	0.25	3.00	0.30%
		7,383,401					

The key assumptions used in calculating the share-based payments are as follows:

a. Black-Scholes valuation methodology was used for all options prior to 2008 and for the 2016 options.

b. The majority of share option awards made since 2011 are performance related and have been modeled using the Monte-Carlo methodology. The options granted on January 31, 2013 and December 18, 2013 at an exercise price of 20 pence respectively, and 16,667 of the unapproved options granted on June 23, 2014 are not performance related.

c. Figures in the range of 18-134% have been used for expected volatility. This has been derived from historic share price performance, weighted to exclude periods of unusually high volatility.

d. Expected dividend yield is nil, consistent with the Directors' view that the Group's business model is to generate value through capital growth rather than the payment of dividends.

e. The risk free rate is equal to the prevailing UK Gilts rate at grant date that most closely matches the expected term of the grant.

f. Share options are assumed to be exercised immediately on vesting.

g. The fair value of the options awarded on May 10, 2012 is the average of the fair values calculated per possible vesting installment.

23. Fixed assets purchase commitments

At January 31, 2017, the Group had no capital commitments (January 31, 2016: nil).

24. Leasing and other commitments

The Group's total commitments under non-cancelable operating leases are as follows:

	Land & Buildings	
	January 31, 2017 £000s	January 31, 2016 £000s
Leases which expire		
Not later than one year	**88**	97
Later than one year and not later than five years	**122**	194
	210	**291**

On February 17, 2017, following the year end, the Group signed a ten year lease for new UK office premises. The total commitment of the new lease over the initial period up until the break clause is £719,579 and the current lease will end on or before August 31, 2017.

In addition to land and buildings, the Group enters into contracts in the normal course of business with contract research organizations to assist in the performance of research and development activities and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancelable contracts and not reflected in the table above.

25. Related party transactions

During the year £nil was paid to Dr. Frank M. Armstrong Consulting Limited, a company controlled by Dr. Frank Armstrong in respect of his fees as Non-Executive Director and Chairman (2016: £18,332). Of this amount £nil was outstanding at the year end (2016: £nil).

Dr. Frank Armstrong is a member of the board of Directors of Juniper Pharmaceuticals Inc. During the year £65,448 (2016: £21,551) was paid to Juniper Pharma Services Limited, a wholly owned subsidiary of Juniper Pharmaceuticals Inc, in respect of clinical manufacturing services. Of this amount £nil was outstanding at the year end (2016: £nil).

See Note 6 'Directors and employees' for details of key management emoluments.

Exhibit 4.23

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

LICENSE AND COLLABORATION AGREEMENT

by and between

SUMMIT (OXFORD) LTD

and

SAREPTA THERAPEUTICS, INC.

Dated as of October 3, 2016

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EXHIBIT & SCHEDULES

Exhibit A	Development Plan and Budget (to be attached upon JSC approval)
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LICENSE AND COLLABORATION AGREEMENT

THIS LICENSE AND COLLABORATION AGREEMENT (this "**Agreement**"), effective as of October 3, 2016 (the "**Effective Date**"), is entered into by and between Summit (Oxford) Ltd, a company organized and existing under the laws of England and Wales ("**Summit**") and, Sarepta Therapeutics, Inc., a corporation organized and existing under the laws of Delaware ("**Sarepta**").

RECITALS:

WHEREAS, Summit is a clinical stage biotechnology company that has domain expertise in utrophin modulation, owns or controls certain key intellectual property relating to utrophin modulator compounds and is developing proprietary therapeutic products in the Field (as defined below);

WHEREAS, Sarepta is a pharmaceutical company that has expertise and capabilities in researching, developing and marketing RNA-based technologies for DMD (as defined below) and infectious diseases;

WHEREAS, the Parties desire to collaborate to discover and develop first-in-class and best-in-class utrophin modulators for DMD and BMD (as defined below);

WHEREAS, Sarepta desires to develop and commercialize such utrophin modulators in the Sarepta Territory (as defined below); and

WHEREAS, Summit desires to develop and commercialize such utrophin modulators in the Summit Territory (as defined below).

NOW, **THEREFORE**, in consideration of the foregoing premises and the mutual covenants herein contained, the Parties hereby agree as follows:

1. DEFINITIONS

Unless specifically set forth to the contrary herein, the following terms, whether used in the singular or plural, shall have the respective meanings set forth below:

1.1. "**Act in Concert**" has the meaning ascribed thereto in the City Code.

1.2. "**Affiliate**" means, with respect to a Person, any other Person which controls, is controlled by, or is under common control with the applicable Person. For purposes of this definition, "control" shall mean: (a) in the case of corporate entities, direct or indirect ownership of at least fifty percent (50%) of the stock or shares (or such lesser percentage which is the maximum allowed to be owned by a foreign corporation in a particular jurisdiction) entitled to vote for the election of directors, or otherwise having the power to control or direct the affairs of such Person; and (b) in the case of non-corporate entities, direct or indirect ownership of at least fifty percent (50%) of the equity interest or the power to direct the management and policies of such non-corporate entities.

1.3. "**API Bulk Drug Substance**" means a Collaboration Compound in bulk form manufactured for use as an active pharmaceutical ingredient.

1.4. "**Benzoxazole Collaboration Compound**" means ezutromid [**].

1.5. "**Benzoxazole Licensed Product**" means each product comprising or containing a Benzoxazole Collaboration Compound; [**].

1.6. "**BMD**" means Becker Muscular Dystrophy.

1.7. "**Calendar Quarter**" means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31 of each Calendar Year; provided that (a) the first Calendar Quarter of the Term shall begin on the Effective Date and end on the December 31st thereafter and the last Calendar Quarter of the Term shall end on the last day of the Term and (b) the first Calendar Quarter of a Royalty Term for a Licensed Product in a country shall begin on the First Commercial Sale of a Licensed Product in such country and end on the first to occur of March 31, June 30, September 30 or December 31 thereafter and the last Calendar Quarter of a Royalty Term shall end on the last day of such Royalty Term in such country.

1.8. "**Calendar Year**" means each successive period of twelve (12) months commencing on January 1 and ending on December 31; provided that (a) the first Calendar Year of the Term shall begin on the Effective Date and end on the first December 31 thereafter and the last Calendar Year of the Term shall end on the last day of the Term and (b) the first Calendar Year of a Royalty Term for a Licensed Product in a country shall begin on the First Commercial Sale of a Licensed Product in such country and end on the first December 31 thereafter and the last Calendar Year of the Term shall end on the last day of such Royalty Term in such country.

1.9. "**Change of Control**" shall occur with respect to a Party if: (a) any Third Party acquires directly or indirectly the beneficial ownership of any voting security of such Party, or if the percentage ownership of such Third Party in the voting securities of such Party is increased through stock redemption, cancellation or other recapitalization, and immediately after such acquisition or increase such Third Party is, directly or indirectly, the beneficial owner of voting securities representing more than fifty percent (50%) of the total voting power of all of the then outstanding voting securities of such Party; (b) a merger, consolidation, recapitalization or reorganization of such Party is consummated, other than any such transaction, which would result in shareholders or equity holders of such Party immediately prior to such transaction, owning at least fifty percent (50%) of the outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; (c) the shareholders or equity holders of such Party approve a plan of complete liquidation of such Party, or an agreement for the sale or disposition by such Party of all or substantially all of such Party's assets, other than pursuant to the transaction described above or to an Affiliate; or (d) the sale or transfer to a Third Party of all or substantially all of such Party's consolidated assets taken as a whole.

1.10. "**CIS**" means Armenia, Azerbaijan, Belarus, Kazakhstan, Kyrgyzstan, Moldova, Russia, Tajikistan, Turkmenistan, Ukraine and Uzbekistan.

1.11. "**City Code**" means the City Code on Takeovers and Mergers as promulgated from time to time by the London Panel on Takeovers and Mergers.

1.12. "**Clinical Study**" means a Phase 1 Study, Phase 2 Study, Pivotal Clinical Study or Post-Approval Study, as applicable.

1.13. "**Collaboration**" means the collaboration of the Parties in the Development, Manufacture and Commercialization of Collaboration Compounds and Licensed Products under this Agreement.

1.14. "**Collaboration Compound**" means each Benzoxazole Collaboration Compound and each Next Generation Collaboration Compound.

1.15. "**Collaboration Know-How**" means any Know-How or interest therein that is invented, developed or generated on or after the Effective Date jointly by or on behalf of (a) Sarepta or its Related Parties or Third Parties on behalf of or pursuant to contracts with Sarepta or its Related Parties, on the one hand and (b) Summit or its Related Parties or Third Parties on behalf of or pursuant to contracts with Summit or its Related Parties, on the other hand, in the Development, Manufacture or Commercialization of Collaboration Compounds or Licensed Products.

1.16. "**Collaboration Patent Rights**" means any Patent Rights that Cover the Collaboration Know-How.

1.17. "**Collaboration Technology**" means, collectively, Collaboration Know-How and Collaboration Patent Rights.

1.18. "**Commercialization**" or "**Commercialize**" means any and all activities directed to marketing, promoting, distributing, importing, exporting, offering to sell or selling a product and activities directed to obtaining pricing and reimbursement approvals, as applicable.

1.19. "**Commercially Reasonable Efforts**" means (a) with respect to each Party's obligations under this Agreement that relate to a Licensed Product (including Development, Manufacture or Commercialization obligations), those efforts reasonably used by an entity in the biotechnology/pharmaceutical industry of similar resources and expertise as such Party, for such similar entity's own products (including internally developed, acquired and in-licensed products) of similar market potential at a similar stage in development or product life, taking into account all relevant factors, including (i) issues of safety, tolerability and efficacy, (ii) product profile, (iii) difficulty in and costs of developing or manufacturing the Licensed Product, (iv) competitiveness of the Licensed Product and competitive products (but without considering competitive products Controlled by the Party to which the efforts obligation applies) in the marketplace, (v) the extent of market exclusivity, (vi) the patent or other proprietary position of the Licensed Products, (vii) Third Party intellectual property rights, (viii) the regulatory structure

involved and (ix) the potential profitability of the Licensed Products; and (b) with respect to such Party's other obligations under this Agreement, the carrying out of such obligations in a diligent, expeditious and sustained manner using efforts and resources, including reasonably necessary personnel and financial resources, that biopharmaceutical companies of comparable size and resources typically devote to similar tasks under similar circumstances.

1.20. "**Competing Product**" means [**].

1.21. "**Confidential Information**" means any and all confidential or proprietary information and data, including Summit Know-How, Sarepta Know-How and Collaboration Know-How and all other scientific, pre-clinical, clinical, regulatory, manufacturing, marketing, financial and commercial information or data, whether communicated in writing or orally or by any other method, that is provided by one Party to the other Party in connection with this Agreement. Summit Know-How is the Confidential Information of Summit. Sarepta Know-How is the Confidential Information of Sarepta. Collaboration Know-How and the terms of this Agreement are the Confidential Information of both Parties.

1.22. "**Control**," "**Controls**" or "**Controlled by**" means, with respect to any Know-How, Patent Rights or other intellectual property rights, the possession of (whether by ownership or license, other than pursuant to this Agreement), and the ability of a Person or its Affiliates to assign, transfer, or grant access to, or to grant a license or sublicense of, such item or right as provided for herein without violating the terms of any agreement or other arrangement with any Third Party existing at the time such Person would be required hereunder to assign, transfer or grant another Person such access or license or sublicense. Notwithstanding the foregoing, Know-How, Patent Rights or other intellectual property rights will not be "Controlled" by a Party under this Agreement by virtue of such Know-How, Patent Rights or other intellectual property rights being owned or in-licensed by a Third Party at the time that such Third Party becomes an Affiliate of such Party after the Effective Date as a result of such Party being acquired by such Third Party (whether by merger, stock purchase or purchase of assets).

1.23. "**Cost of Goods**" means, with respect to API Bulk Drug Substance, Finished Drug Product or placebo, as the case may be, Manufactured under this Agreement, the reasonable internal and external costs of a Party or any of its Related Parties incurred in Manufacturing such API Bulk Drug Substance, Finished Drug Product or placebo, including: (a) to the extent that such API Bulk Drug Substance, Finished Drug Product or placebo is Manufactured by a Party or any of its Related Parties, direct material (including shipping) and direct labor costs, plus a reasonable allocation of Manufacturing plant overhead (including start-up costs and depreciation) and a reasonable allocation of the costs of failed batches, to be further described in the Supply Agreement, but excluding corporate and administrative overhead and costs associated with excess capacity, all determined in accordance with the books and records of the applicable Party or its Related Party(ies) maintained in accordance with GAAP, consistently applied and (b) to the extent that such API Bulk Drug Substance, Finished Drug Product or placebo is Manufactured by a Third Party manufacturer, the actual fees paid by a Party or any of its Related Parties to the Third Party for the Manufacture, supply, packaging, labeling and

shipping of such API Bulk Drug Substance, Finished Drug Product or placebo, and any reasonable Out-of-Pocket Costs and direct labor costs actually incurred by such Party or any of its Related Parties in managing or overseeing the Third Party relationship, all determined in accordance with the books and records of the applicable Party or its Related Party(ies) maintained in accordance with GAAP, consistently applied.

1.24. "**Cover**," "**Covering**" or "**Covers**" means, as to a Licensed Product and Patent Rights, that, in the absence of a license granted under, or ownership of, such Patent Rights, the manufacture, use, offer for sale, sale or importation of such Licensed Product would infringe such Patent Rights assuming the validity and enforceability thereof or, as to a pending claim included in such Patent Rights, the manufacture, use, offer for sale, sale or importation of such Licensed Product would infringe such Patent Rights if such pending claim were to issue in an issued patent.

1.25. "**CPI**" shall mean the Consumer Price Index – Urban Wage Earners and Clerical Workers, U.S. City Average, All Items, 1982-84 = 100, published by the United States Department of Labor, Bureau of Labor Statistics (or its successor equivalent index) in the United States.

1.26. "**Development**," "**Developing**" or "**Develop**" means, with respect to a Collaboration Compound or Licensed Product, all activities relating to the discovery, evaluation, research and preclinical, non-clinical and clinical development of such Collaboration Compound or Licensed Product prior to or after receiving Regulatory Approval, and all regulatory activities in support of obtaining Regulatory Approval other than activities directed to obtaining pricing and reimbursement approvals.

1.27. "**Development Candidate**" means any Next Generation Collaboration Compound that is designated by the JSC as a Development Candidate in accordance with Section 2.3.1, or included in the Collaboration, following Sarepta's exercise of a Declined NG Candidate Option in accordance with Section 2.3.3(c).

1.28. "**Development Costs**" means the Out-of-Pocket Costs and FTE Costs (or such other measure of costs as may be specified in the Development Plan) incurred by either Party or any of its Related Parties in Developing Collaboration Compounds and Licensed Products in conducting activities contemplated by the then-current Development Plan (including any such costs incurred in connection with preparing and submitting any IND to applicable Regulatory Authorities), in accordance with this Agreement and determined from the books and records of such Party and its Affiliates maintained in accordance with GAAP; provided that such activities and costs are consistent with the then-current Development Plan and budget contained therein. Development Costs exclude all Third Party License Payments and all payments due under Summit's existing grant agreements with the Muscular Dystrophy Association, Inc. and the Duchenne Partner's Fund. Notwithstanding the foregoing, Development Costs will not include any costs incurred in connection with regulatory activities in support of obtaining any Regulatory Approval for any Collaboration Compound or Licensed Product, including the cost of preparing and submitting any NDA with respect to a Collaboration Compound or Licensed Product or interacting with Regulatory Authorities, which costs shall be borne by the Parties as set forth in Section 3.3.

1.29. "**Development Plan**" means the written work plan, timetable and budget for the Parties' Licensed Product Development efforts, as approved by the JSC and amended from time to time in accordance with this Agreement.

1.30. "**DMD**" means Duchenne Muscular Dystrophy.

1.31. "**EMA**" means the European Medicines Agency and any successor governmental authority having substantially the same function.

1.32. "**EU**" means the European Union, as its membership may be altered from time to time, and any successor thereto; provided, however, that, for purposes of this Agreement, the United Kingdom shall be considered a part of the EU irrespective of its membership status.

1.33. "**Exchange Act**" means the Securities Exchange Act of 1934, as amended, and the rules and regulations of the SEC promulgated thereunder.

1.34. "**Existing Summit In-Licenses**" means the agreement set forth on Schedule 1.34.

1.35. "**FDA**" means the United States Food and Drug Administration and any successor governmental authority having substantially the same function.

1.36. "**FDCA**" means the United States Federal Food, Drug, and Cosmetic Act of 1938, as amended from time to time, and the regulations and guidelines promulgated thereunder.

1.37. "**Field**" means all therapeutic and commercial applications of utrophin modulators in humans for any indication, particularly (but without limitation) for therapy of the dystrophinopathies DMD and BMD.

1.38. "**Finished Drug Product**" means the finished product formulation of a Licensed Product, containing API Bulk Drug Substance, filled into unit packages for final labeling and packaging, and as finally labeled and packaged in a form ready for administration.

1.39. "**First Commercial Sale**" means, with respect to a Licensed Product in a country, the first sale for end use or consumption of such Licensed Product in such country after all Regulatory Approvals legally required for such sale have been granted by the Regulatory Authority of such country.

1.40. "**FTE**" means [**] hours per year of work devoted to or in support of the Development or Manufacture of a Licensed Product that is carried out by one or more qualified scientific, technical or operational management employees of a Party or its Affiliates.

1.41. "**FTE Cost**" means, for any period, the FTE Rate multiplied by the number of FTEs in such period.

1.42. "**FTE Rate**" means [**] per year per FTE, increased annually beginning on January 1, 2017 and thereafter on January 1 of each succeeding year by the percentage increase in the CPI as of December 31 of the then most recently ended calendar year over the level of the CPI on December 31, 2015.

1.43. "**GAAP**" means United States Generally Accepted Accounting Principles (GAAP), or International Financial Reporting Standards (IFRS) if required in lieu of GAAP for a public company filing financial reports with the U.S. Securities and Exchange Commission, in each case, as then current at the relevant time and as consistently applied by the applicable Party and its Affiliates.

1.44. "**Generic Competition**" means, with respect to a Licensed Product in any country in the Sarepta Territory in a given Calendar Quarter, that, during such Calendar Quarter, (a) one or more Generic Products are commercially available in such country and (b) such Generic Products achieve [**] or more of the unit-based aggregate market share in such country of the Licensed Product and such Generic Product during such Calendar Quarter as determined by IMS Health data (or data from an alternative data source that the Parties mutually agree to use).

1.45. "**Generic Product**" for a given country means a pharmaceutical product that (a) is sold by a Person that is not a Related Party of Sarepta and that has not been granted authorization by Sarepta or any of its Related Parties to make such sales, (b) contains the same active ingredient(s) as are contained in a Licensed Product and (c) is approved by the Regulatory Authority pursuant to an abbreviated approval process that relies, in whole or in part, on such Regulatory Authority's previous grant of marketing authorization for a Licensed Product, or on the safety or efficacy data submitted in support of such marketing authorization.

1.46. "**Governmental Authority**" means any applicable government authority, court, tribunal, arbitrator, agency, department, legislative body, commission or other instrumentality of (a) any government of any country or territory, (b) any nation, state, province, county, city or other political subdivision thereof or (c) any arbitral or supranational body.

1.47. "**ICH**" means International Conference on Harmonization.

1.48. "**IMPD**" means an Investigational Medicinal Product Dossier.

1.49. "**IND**" means an Investigational New Drug application, Clinical Trial Application or similar application or submission for approval to conduct human clinical investigations filed with or submitted to a Regulatory Authority in conformance with the requirements of such Regulatory Authority.

1.50. "**Indication**" means any human disease, condition or syndrome, or sign or symptom of, or associated with, a human disease or condition.

1.51. "**Initiation**" means, with respect to a Licensed Product and a Clinical Study, the first receipt by the first human subject in such Clinical Study of his or her first dosing with such Licensed Product in such Clinical Study.

1.52. "**In-Licenses**" means, collectively, the Summit In-Licenses and the Sarepta In-Licenses.

1.53. "**Joint Steering Committee**" or "**JSC**" means the joint steering committee as more fully described in Section 5.1.

1.54. "**Know-How**" means all chemical or biological materials and other tangible materials, inventions, improvements, practices, discoveries, developments, data, information, technology, methods, protocols, formulas, knowledge, know-how, trade secrets, processes, assays, skills, experience, techniques and results of experimentation and testing, including pharmacological, toxicological and pre-clinical and clinical data and analytical and quality control data; provided, however, excluding in any event any Patent Rights.

1.55. "**Laws**" means all applicable laws, statutes, rules, regulations, orders, judgments, injunctions, ordinances or other pronouncements having the binding effect of law of any Governmental Authority.

1.56. "**Licensed Product**" means (a) each Benzoxazole Licensed Product and (b) each Next Generation Product.

1.57. "**Major European Country**" means [**].

1.58. "**Major Option Country**" means [**].

1.59. "**Manufacturing**" or "**Manufacture**" means, as applicable, all activities associated with the production, manufacture, process of formulating, processing, filling, finishing, packaging, labeling, shipping, importing and storage of Licensed Products (including API Bulk Drug Substance, Finished Drug Product and placebo), including process development, process validation, stability testing, manufacturing scale-up, pre-clinical, clinical and commercial manufacture and analytical development, product characterization, quality assurance and quality control development, testing and release.

1.60. "**NDA**" means a New Drug Application, Marketing Authorization Application or similar application or submission filed with a Regulatory Authority in a country or group of countries to obtain marketing approval for a biological, pharmaceutical or other therapeutic or prophylactic product in that country or in that group of countries.

1.61. "**Net Sales**" means the aggregate gross invoiced sales prices from sales of all units of Licensed Products sold by Sarepta and its Related Parties to independent Third Parties after deducting, if not previously deducted, from the amount invoiced:

(a) [**];

(b) [**];

(c) [**];

(d) [**];

(e) [**]; and

(f) [**].

Such amounts shall be determined from the books and records of Sarepta or its Related Parties, maintained in accordance with GAAP.

In the case of any sale or other disposal for value, such as barter or counter-trade, of a Licensed Product, or part thereof, other than in an arm's length transaction exclusively for cash, Net Sales shall be calculated as above on the value of the non-cash consideration received or, if higher, the fair market price of the Licensed Product in the country of sale or disposal, as determined in accordance with GAAP.

Notwithstanding the foregoing, the following will not be included in Net Sales: [**].

In the event that a Licensed Product is sold in the form of a combination product containing one or more active pharmaceutical ingredients in addition to such Licensed Product, Net Sales of such combination product shall be adjusted by [**].

1.62. "**Next Generation Collaboration Compound**" means any small molecule utrophin modulator other than a Benzoxazole Collaboration Compound that is identified or developed (a) prior to or on the Effective Date by Summit or any of its Affiliates or (b) at any time in the conduct of the Collaboration by or on behalf of either Party or any of its Affiliates or any of its or their Sublicensees (for Sarepta) or licensees (for Summit). Each "Next Generation Collaboration Compound" includes [**].

1.63. "**Next Generation Product**" means each product comprising or containing a Next Generation Collaboration Compound; provided, however, products that contain different dosages and formulations of a product, but utilize the same specific Next Generation Collaboration Compound, shall not be considered distinct Next Generation Products.

1.64. "**Option Data Package**" means, with respect to a Declined NG Development Candidate, the information and materials set forth on Schedule 1.64.

1.65. "**Option Territory**" means [**].

1.66. "**Out-of-Pocket Costs**" means, with respect to certain activities hereunder, direct expenses paid or payable by either Party or its Affiliates to Third Parties and specifically identifiable and incurred to conduct such activities for a Collaboration Compound or Licensed Product, including payments to contract personnel.

1.67. "**Party**" means Sarepta or Summit.

1.68. "**Patent Rights**" means (a) all issued patents (including extensions, restorations by existing or future extension or registration mechanism, including patent term adjustments, patent term extension, supplemental protection certificates or the equivalent thereof,

substitutions, confirmations, re-registrations, re-examinations, and patents of addition), (b) patent applications (including all provisional applications, substitutions, requests for continuation, continuations, continuations-in-part, divisionals and renewals), (c) inventor's certificates and (d) and all equivalents of the foregoing in any country of the world.

1.69. "**Person**" shall mean any natural person, corporation, unincorporated organization, partnership, association, sole proprietorship joint stock company, joint venture, limited liability company, trust or government, or any agency or political subdivision of any government, or any other similar entity.

1.70. "**Phase 1 Study**" means a study in humans which provides for the introduction into humans of a product, conducted in healthy volunteers or patients, to obtain initial information on product safety, tolerability, pharmacological activity or pharmacokinetics, as more fully defined in 21 C.F.R. § 312.21(a) (or the equivalent thereof outside the United States).

1.71. "**Phase 2 Study**" means a study in humans of the safety, dose ranging or efficacy of a product, as further defined in 21 C.F.R. § 312.21(b) (or the equivalent thereof outside the United States).

1.72. "**Pivotal Clinical Study**" means any human clinical study of a product that, if pre-specified primary endpoints are met, would demonstrate the safety and efficacy of such product as required to support the Regulatory Approval of such product in an applicable country or territory.

1.73. "**Post-Approval Study**" means a non-human or human clinical study of a product initiated after receipt of Regulatory Approval for such product in a country or territory.

1.74. "**Product Trademark(s)**" means the Trademarks for use in connection with the distribution, marketing, promotion and sale of the Licensed Product(s). Product Trademarks specifically excludes the corporate names and logos of the Parties and their Affiliates. Product Trademarks include both the Summit Trademarks and the Sarepta Trademarks.

1.75. "**Regulatory Approval**" means any and all approvals, licenses, registrations or authorizations of any Regulatory Authority that are necessary for the marketing and sale of a product in a country or group of countries (including all pricing and reimbursement approvals, if required for sale of a product in such country or group of countries).

1.76. "**Regulatory Authority**" means any applicable government regulatory authority involved in granting approvals for the Development, Manufacturing or Commercialization of Licensed Products, including the FDA and the EMA.

1.77. "**Regulatory Exclusivity**" means, with respect to a Licensed Product in a country, any exclusive marketing right, data protection or other exclusive right, other than a Patent Right, conferred by any Regulatory Authority with respect to such Licensed Product in such country, including new drug exclusivity, new indication or use exclusivity, pediatric exclusivity or orphan drug exclusivity.

1.78. "**Related Party**" means a Party's Affiliates, permitted Sublicensees and, with respect to Summit in the Summit Territory, licensees, but, with respect to Sarepta, excluding Third Party Distributors.

1.79. "**Royalty Term**" means, on a Licensed Product-by-Licensed Product and country-by-country basis, the period commencing with the First Commercial Sale of such Licensed Product in such country and continuing until [**].

1.80. "**Sarepta In-License**" means any agreement entered into between Sarepta or its Affiliates and one or more Third Parties pursuant to which Sarepta or such Affiliate Controls Patent Rights or Know-How that are reasonably necessary or useful for Summit to Develop, Manufacture or Commercialize Collaboration Compounds or Licensed Products in the Field in the Summit Territory.

1.81. "**Sarepta Know-How**" means Know-How, other than Collaboration Know-How, that is Controlled by Sarepta or its Affiliates during the Term and is reasonably necessary or useful for Summit to Develop, Commercialize or Manufacture Collaboration Compounds or Licensed Products in the Field in the Summit Territory.

1.82. "**Sarepta Patent Rights**" means those Patent Rights, other than Collaboration Patent Rights, that are Controlled by Sarepta or its Affiliates during the Term and are reasonably necessary or useful to Develop, Commercialize or Manufacture Collaboration Compounds or Licensed Products in the Field in the Summit Territory.

1.83. "**Sarepta Technology**" means, collectively, Sarepta Know-How, Sarepta Patent Rights and Sarepta's interest in the Collaboration Technology.

1.84. "**Sarepta Territory**" means (a) the EU, the CIS, Switzerland, Norway, Iceland and Turkey and (b) if Sarepta exercises the Territory Expansion Option pursuant to Section 4.2, the Option Territory.

1.85. "**Serious Adverse Event**" means any adverse event that (a) results in death, (b) is life threatening, (c) requires inpatient hospitalization or prolongation of existing hospitalization, (d) results in persistent or significant disability or incapacity, (e) is a congenital anomaly or birth defect or (f) based upon appropriate medical judgment is considered an important medical event that may jeopardize the patient or subject and may require medical or surgical intervention to prevent one or more outcomes listed in this definition.

1.86. "**Sublicensee**" means a Third Party to whom a Party grants a sublicense under any Summit Technology or Sarepta Technology, as the case may be, to Develop, Manufacture or Commercialize a Licensed Product in the Field pursuant to Section 7.1.2 or Section 7.2.2 (as applicable).

1.87. "**Summit In-License**" means (a) each Existing Summit In-License and (b) any agreement entered into on or following the Effective Date between Summit or its Affiliates and one or more Third Parties pursuant to which Summit or such Affiliate Controls Patent Rights or Know-How that are reasonably necessary or useful for Sarepta to Develop, Manufacture or Commercialize Collaboration Compounds or Licensed Products in the Field in the Sarepta Territory.

1.88. "**Summit Know-How**" means Know-How, other than Collaboration Know-How, that is Controlled by Summit or its Affiliates during the Term that is reasonably necessary or useful for Sarepta to Develop, Manufacture or Commercialize Collaboration Compounds or Licensed Products in the Field in the Sarepta Territory.

1.89. "**Summit Patent Rights**" means those Patent Rights, other than Collaboration Patent Rights, that are Controlled by Summit or its Affiliates during the Term that are reasonably necessary or useful to Develop, Manufacture or Commercialize Collaboration Compounds or Licensed Products in the Field in the Sarepta Territory, including the Patent Rights identified on Schedule 1.89.

1.90. "**Summit Technology**" means, collectively, Summit Know-How, Summit Patent Rights and Summit's interest in the Collaboration Technology.

1.91. "**Summit Territory**" means all countries and territories of the world other than the Sarepta Territory.

1.92. "**Territory**" means (a) with respect to Summit, the Summit Territory and (b) with respect to Sarepta, the Sarepta Territory.

1.93. "**Third Party**" means an entity other than a Party and its Affiliates.

1.94. "**Third Party Distributor**" means any Third Party appointed by Sarepta or any of its Related Parties to distribute, market and sell any Licensed Product, with or without packaging rights, in one or more countries in the Sarepta Territory, in circumstances where such Third Party purchases its requirements of Licensed Product from Sarepta or its Related Parties for resale but does not (a) make any royalty or profit share payment to Sarepta or its Related Parties with respect to its resale of such Licensed Product or (b) assume primary responsibility for advertising, promotion and sales force activities for such Licensed Product in such countries.

1.95. "**Third Party License Payment**" means royalties, upfront fees, milestones or other amounts payable under an In-License, excluding sponsored research funding payments made to Third Parties for Development activities included in the Development Plan.

1.96. "**Trademark**" means any trademark, trade name, service mark, service name, brand, domain name, trade dress, logo, slogan or other indicia of origin or ownership, including the goodwill and activities associated with each of the foregoing.

1.97. "**United States**" or "**U.S.**" means the United States of America and its territories, possessions and commonwealths.

1.98. "**University of Oxford Option Agreement**" means the agreement dated November 22, 2013 entered into by and between Summit Corporation PLC, the Chancellor, Masters and Scholars of the University of Oxford ("**Oxford**") and Isis Innovation Limited ("**Isis**"), as amended by the Variation Agreement dated November 16, 2015.

1.99. "**Valid Claim**" means a claim of: (a) an issued and unexpired patent, which claim has not lapsed or been dedicated to the public, withdrawn, cancelled, abandoned, disclaimed, revoked or held unpatentable, unenforceable or invalid by an unappealable decision of a court or other governmental agency of competent jurisdiction, or has not been appealed within the time allowed for appeal, or by an appealed decision of a court or other governmental agency of competent jurisdiction where the appeal has been pending for more than two (2) years (unless and until such decision is subsequently overturned on appeal) and which has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue, re-examination or disclaimer or otherwise or (b) a patent application that has been pending less than [**] from the date of filing of the earliest patent application from which such patent application claims priority, which claim has not been cancelled, withdrawn or abandoned or finally rejected by an administrative agency action from which no appeal can be taken.

1.100. **Additional Definitions**. Each of the following definitions is set forth in the section of this Agreement indicated below:

DEFINITION:	SECTION:
Acquiring Party	7.7.1
Additional Study	2.2.3(c)
Agreement	Preamble
Back-Up Source	6.4
Bankrupt Party	7.4
Bankruptcy Code	7.4
Bulk Drug Product	Schedule 6.2
cGMP	6.1
CEO	14.1
Clinical Supply Agreement	6.2
Collaboration Manager	5.2
Commercial Supply Agreement	6.2
Competitive Infringement	12.3.1
Contracting Party	5.5
Declined NG Candidate Option	2.3.3(a)
Declined NG Candidate Development Costs	2.3.3(d)(ii)
Declined NG Development Candidate	2.3.1
Defense Action	12.3.1
Development Buy-In	2.2.3(d)
Effective Date	Preamble
Global Branding Strategy	4.4.1
Indemnitee	11.4
Isis	1.98
Losses	11.1

DEFINITION:	SECTION:
Manufacturing Arbitration Draft	6.3.1
Neutral Expert	6.3.2
Non-Bankrupt Party	7.4
Non-Proposing Party	2.2.3(c)
Opt-In Study	2.3.3(b)
Option Commencement Notice	2.3.3(b)
Option Exercise Notice	2.3.3(c)
Option Period	2.3.3(c)
Oxford	1.98
Pharmacovigilance Agreement	3.4
Post-Development Buy-In	2.2.3(e)
Promotional Materials	4.4.2
Proposing Party	2.2.3(c)
Safety Termination	13.2.3
Sarepta	Preamble
Sarepta Indemnitees	11.2
Sarepta Territory Commercialization Plan	4.3.1
Sarepta Trademarks	12.8.2
Second Source	6.4
SPC	12.3.4
Standstill Parties	14.1
Standstill Period	14.1
Summit	Preamble
Summit Indemnitees	11.1
Summit Trademarks	12.8.2
Supply Agreement	6.2
Technology Transfer	6.5
Territory Expansion Option	4.2
Territory Expansion Option Exercise Date	4.2
Territory Expansion Option Fee	4.2
Term	13.1
Terminated Countries	13.3.2
Terminated Licensed Product	13.3.1
Third Party Collaboration Agreement	5.5
Third Party Partner	5.5

2. DEVELOPMENT COLLABORATION

2.1. **Overview**. Prior to the Effective Date, Summit has been engaged in the Development of Licensed Products. Under this Agreement, the Parties will collaborate in the further Development of Licensed Products in accordance with the Development Plan.

2.2. **Development Plan; Amendments; Development Costs**.

2.2.1. **Development Plan**. The Development of Collaboration Compounds and Licensed Products shall be governed by the Development Plan, and the Parties

may not Develop any Collaboration Compound or Licensed Product other than in accordance with the Development Plan or as set forth in Section 2.2.3(c) or Section 2.3. Each Party shall use Commercially Reasonable Efforts to conduct all their Development activities relating to Licensed Products in accordance with the Development Plan. Summit shall prepare the initial draft of the initial Development Plan for review and approval by the JSC. The JSC shall be responsible for approving the initial Development Plan. Unless otherwise approved by the JSC, the Development Plan shall include in reasonable detail (a) all Development activities reasonably anticipated to be undertaken by each Party, (b) the endpoints for all Clinical Studies contemplated by such plan, (c) which Clinical Study is intended to be a Pivotal Clinical Study, (d) all regulatory activities and interactions anticipated to be conducted by each Party in support of Regulatory Approval of each Licensed Product, including all planned regulatory filings to be submitted in connection with such approvals, (e) a good faith non-binding estimate of the dates on which the Parties expect to achieve each milestone event set forth in TABLE 8.2.1, and, if applicable, TABLE 8.2.2 and TABLE 8.2.3 and (f) a budget for all Development Costs. The JSC shall approve the initial Development Plan within ninety (90) days after the Effective Date in accordance with Section 5.3.1(a), and once the JSC approves such initial Development Plan, it will be attached to this Agreement as Exhibit A.

2.2.2. **Amendments**. Following the JSC's approval of the initial Development Plan, it shall review the Development Plan not less frequently than annually and shall develop detailed and specific Development Plan updates, each of which shall update and include annual Development budgets for the following Calendar Year until the completion of Licensed Product Development activities. The Parties may also develop and submit to the JSC from time to time other proposed substantive amendments to the Development Plan. In addition, upon approval of any Additional Study by the JSC pursuant to Section 5.3.1(d) and the Non-Proposing Party's agreement to co-fund such study, the JSC will amend the Development Plan to include such Additional Study. Further, upon designation of such Next Generation Collaboration Compound as a Development Candidate pursuant to Section 5.3.1(i), the Parties will develop and submit to the JSC an amendment to the Development Plan that includes the proposed Development activities with respect to such Next Generation Collaboration Compound. The JSC shall review such proposed amendments and may approve such proposed amendments or any other proposed amendments that the JSC may consider from time to time in its discretion and, upon such approval by the JSC, the Development Plan shall be amended accordingly. Amendments and updates to the Development Plan, including any budgets contained in the Development Plan, shall not be effective without the approval of the JSC.

2.2.3. Development Costs.

(a) Responsibility for Costs.

(i) During the period beginning on the Effective Date and ending on December 31, 2017, subject to Section 2.2.3(a)(iii), Summit shall be solely responsible for all Development Costs; *provided*, *however*, that, if Sarepta incurs Development Costs in excess of one hundred ten percent (110%) of the Development Costs budgeted for activities assigned to Sarepta in the budget of the then-current version of the Development Plan, then Sarepta shall be solely responsible for such excess costs unless Summit agrees in writing to assume them.

(ii) Beginning on January 1, 2018, subject to Section 2.2.3(a)(iii) and Section 2.2.3(c), Summit shall pay fifty-five percent (55%) and Sarepta shall pay forty-five percent (45%) of all Development Costs that are within one hundred ten percent (110%) of the Development Costs budgeted for activities included in the budget of the then-current version of the Development Plan. Any Development Costs in excess of one hundred ten percent (110%) of such budgeted Development Costs shall be borne solely by the Party incurring such costs unless such Party has received the other Party's written approval to share such excess costs.

(iii) If Sarepta exercises the Territory Expansion Option pursuant to Section 4.2, then, from and after the Territory Expansion Option Exercise Date, Sarepta shall be solely responsible for all Development Costs specifically related to the Option Territory; provided, however, that, if Summit incurs Development Costs specifically related to the Option Territory in excess of one hundred ten percent (110%) of the Development Costs budgeted for activities assigned to Summit in the budget of the then-current version of the Development Plan, then Summit shall be solely responsible for such excess costs unless Sarepta agrees in writing to assume them.

(iv) In each case contemplated by Sections 2.2.3(a)(i) – 2.2.3(a)(iii), Development Costs shall initially be borne by the Party incurring the cost or expense, subject to reimbursement as provided in Section 2.2.3(b). Each Party shall calculate and maintain records of Development Costs incurred by it in accordance with procedures to be established by the JSC pursuant to Section 5.3.1(e).

(b) Development Cost Reports. Within fifteen (15) business days following the beginning of the last month of each Calendar Quarter, each Party shall prepare and deliver to the JSC a quarterly report detailing its and its Affiliates' Development Costs (i) incurred during the first two (2) months of such Calendar Quarter, (ii) estimated to be incurred during

the last month of such Calendar Quarter and (iii) actually incurred in the last month of the immediately preceding Calendar Quarter, in each case, ((i)-(iii)), that are required to be shared pursuant to this Section 2.2.3. Each Party shall submit any supporting information or clarifications reasonably requested by the other Party related to such Development Costs included in such Party's report within ten (10) business days after the other Party's receipt of such request. The Parties, with the assistance of the JSC, shall conduct a reconciliation of Development Costs for the subject Calendar Quarter within ten (10) business days after receipt of all such supporting information, and an invoice shall be issued to the Party (if any) that has not paid for its full share of the Development Costs for such Calendar Quarter. Such reconciliation shall balance the actual amount of Development Costs incurred during the last month of the immediately preceding Calendar Quarter (to correct for any differences between the estimates and actual amount of such costs) together with the amounts incurred during the first two (2) months of such Calendar Quarter and those estimated to be incurred during the last month of such Calendar Quarter. The paying Party shall pay all amounts payable under any such invoice within forty-five (45) days after its receipt of such invoice.

(c) Additional Studies. If, during the Term, a Party (the "**Proposing Party**") wishes to (i) conduct a Clinical Study or non-clinical study of a Licensed Product that is not (x) contemplated by the initial Development Plan or (y) included in any subsequent version of the Development Plan approved by the JSC or (ii) repeat any Clinical Study or non-clinical study previously conducted under the Development Plan that failed to meet its primary endpoints (each such study in clauses (i) and (ii), an "**Additional Study**"), then (A) the Proposing Party shall first provide the proposed trial design and protocol for such Additional Study to the other Party (the "**Non-Proposing Party**") for review and comment and shall incorporate reasonable comments from the Non-Proposing Party into such Additional Study design and protocol and (B) following such review by the Non-Proposing Party, provide the final proposed design and projected costs of such Additional Study to the JSC for review and approval pursuant to Section 5.3.1(d). The JSC shall approve such Additional Study unless it determines that such Additional Study would be likely to have a material adverse effect on the Development or Commercialization of Licensed Products in the Non-Proposing Party's Territory. After the JSC's review of the Additional Study, the following shall apply:

(i) *JSC Approval of Additional Studies. Co-Funding*. If the JSC approves the Additional Study pursuant to Section 5.3.1(d) and the Non-Proposing Party agrees to co-fund such Additional Study, then the Parties shall amend the Development Plan to

include such Additional Study in accordance with Section 2.2.2, and the costs of such Additional Study shall be included in the Development Cost shared by the Parties in accordance with Section 2.2.3.

(ii) *JSC Approval of Additional Studies. No Co-Funding*. If the JSC approves the Additional Study pursuant to Section 5.3.1(d), but the Non-Proposing Party does not wish to include costs incurred with respect to such proposed Additional Study within the shared Development Cost, then the Proposing Party may proceed with such Additional Study and shall be solely responsible for the conduct and costs of such study. In such case, the Non-Proposing Party would have no rights to use any resulting data in any filings with any Regulatory Authority in the Non-Proposing Party's Territory and would not be granted a right of reference under Section 3.2 with respect to any resulting data, except, in each case, with respect to safety information required to be filed with the applicable Regulatory Authorities, unless and until a Development Buy-In occurs as set forth in Section 2.2.3(d) or a Post-Development Buy-In occurs as set forth in Section 2.2.3(e).

(iii) *Additional Studies Not Approved*. If the JSC does not approve the Additional Study pursuant to Section 5.3.1(d), then the Proposing Party shall not proceed with such Additional Study.

(d) Development Buy-In. At any time prior to the completion of an Additional Study that the Non-Proposing Party declined previously to co-fund, the Non-Proposing Party will have the right to elect by written notice to the Proposing Party to include in the shared Development Costs the costs of such Additional Study (the "**Development Buy-In**"). In such case, (i) the Parties shall share any Development Costs from the day of such notice onward incurred by the Proposing Party to conduct such Additional Study after the Development Buy-In in accordance with Section 2.2.3 and (ii) the Non-Proposing Party shall reimburse the Proposing Party an amount equal to [**] of the costs incurred by the Proposing Party in conducting such Additional Study prior to the day of such notice. Upon any such Development Buy-In, the Parties shall have the rights with respect to such Clinical Studies or studies and the data arising therefrom as set forth in Sections 2.5.3 and 3.2. If the Non-Proposing Party elects a Development Buy-In, then it shall pay to the Proposing Party the amount set forth in the foregoing clause (ii) within forty-five (45) days after the Non-Proposing Party notifies the Proposing Party in writing that the Non-Proposing Party is exercising its right to effect the Development Buy-In pursuant to this Section 2.2.3(d).

(e) Post-Development Buy-In. If the Non-Proposing Party wishes to exercise a buy-in with respect to an Additional Study after the completion of such Additional Study (a "**Post-Development Buy-In**"), then the Non-Proposing Party shall notify the Proposing Party thereof in writing and pay to the Proposing Party for such Additional Study a lump sum payment equal to [**] of the costs that the Proposing Party incurred in conducting such Additional Study. Upon any such Post-Development Buy-In, the Parties shall have the rights with respect to such Clinical Studies or studies and the data arising therefrom as set forth in Sections 2.5.3 and 3.2. If the Non-Proposing Party elects a Post-Development Buy-In, then it shall pay to the Proposing Party the amount set forth in this Section 2.2.3(e) within forty-five (45) days after the Non-Proposing Party notifies the Proposing Party in writing that the Non-Proposing Party is exercising its right to effect the Post-Development Buy-In pursuant to this Section 2.2.3(e).

2.3. Next Generation Products.

2.3.1. **Next Generation Products**. Except as specifically permitted in this Section 2.3.1, neither Summit nor any of its Affiliates shall, itself or with or through any Third Party, engage in any IND-enabling toxicology studies or clinical Development or Commercialization of any Next Generation Collaboration Compound that has not been designated by the JSC as a Development Candidate, or any Next Generation Product containing or comprising a Next Generation Collaboration Compound that is not a Development Candidate. If, during the Term, Summit wishes to conduct any IND-enabling toxicology studies or clinical Development with respect to a Next Generation Collaboration Compound, then Summit will propose such Next Generation Collaboration Compound and the Development activities that it wishes to conduct for such compound to the JSC, and the JSC shall determine whether or not to designate such Next Generation Collaboration Compound as a Development Candidate. If the JSC determines to designate such Next Generation Collaboration Compound as a Development Candidate, then (a) thereafter Sarepta will pay the applicable milestone payment set forth TABLE 8.2.2 as the milestones therein are achieved and (b) the Parties shall amend the Development Plan to include such applicable Development activities for such Next Generation Collaboration Compound in accordance with Section 2.2.2. If the JSC does not designate such Next Generation Collaboration Compound as a Development Candidate (a "**Declined NG Development Candidate**"), then such Declined NG Development Candidate shall no longer be considered a Next Generation Collaboration Compound under this Agreement, and, subject to Section 2.3.2 and Section 2.3.3, Summit and any of its Affiliates shall have the right to engage in further Development, Manufacturing or Commercialization activities with respect to such Declined NG Development Candidate, or any product containing such Declined NG Development Candidate (including through Third Parties), in each case solely for the Summit Territory.

2.3.2. **Declined NG Development Candidate Clinical Limit**. Summit shall not, directly or through an Affiliate or Third Party, concurrently perform Clinical Studies on more than [**].

2.3.3. **Sarepta Option Grant for Declined NG Development Candidates**.

(a) Option Grant. Subject to the terms and conditions of this Agreement, with respect to each Declined NG Development Candidate, Summit hereby grants to Sarepta an exclusive option to include such Declined NG Development Candidate as a Collaboration Compound under this Agreement (each, a "**Declined NG Candidate Option**").

(b) Option Data Package. For each Declined NG Development Candidate, following completion of the first Clinical Study that includes the measurement of a biomarker or other attribute that would show activity of such Declined NG Development Candidate (the "**Opt-In Study**"), Summit shall provide written notice to Sarepta that includes (i) identification of the applicable Declined NG Development Candidate to which the applicable Declined NG Candidate Option applies, (ii) the Option Data Package for such Declined NG Development Candidate and (iii) an estimate of the Declined NG Candidate Development Costs incurred to date (an "**Option Commencement Notice**").

(c) Option Exercise. To exercise a Declined NG Candidate Option, Sarepta must give written notice of exercise of such Declined NG Candidate Option to Summit (an "**Option Exercise Notice**") during the period commencing on the date of Sarepta's receipt of the Option Commencement Notice containing the complete Option Data Package for the applicable Opt-In Study and ending [**] thereafter (each, an "**Option Period**").

(d) Effects of Option Exercise.

(i) *Inclusion as a Development Candidate*. If Sarepta provides written notice to Summit exercising the Declined NG Candidate Option for a Declined NG Development Candidate in accordance with Section 2.3.3(c), then such Declined NG Development Candidate shall become a Development Candidate for purposes of this Agreement (and shall no longer be considered a Declined NG Development Candidate).

(ii) *Cost Reimbursement*. Following Sarepta's exercise of a Declined NG Candidate Option with respect to a Declined NG Development Candidate, Summit shall provide written notice to Sarepta setting forth (A) Summit's Development Costs (for purposes of which definition such Declined NG Development Candidate shall be considered a Collaboration Compound)

incurred prior to the date of Sarepta's exercise of such Declined NG Candidate Option (the "**Declined NG Candidate Development Costs**") and (B) wire transfer instructions for payment of the amounts due to Summit under this Section 2.3.3(d)(ii). Within forty-five (45) days of Sarepta's receipt of such written notice, Sarepta shall pay to Summit an amount equal to [**], in each case, pursuant to the wire transfer instructions provided by Summit in the applicable notice. [**].

(e) Failure to Exercise a Declined NG Candidate Option. If Sarepta either fails to give the Option Exercise Notice with respect to a Declined NG Development Candidate on or before the expiration or termination of the Option Period for such Declined NG Development Candidate or notifies Summit in writing prior to the expiration of such Option Period that Sarepta does not intend to exercise the Declined NG Candidate Option for such Declined NG Development Candidate, then the Declined NG Candidate Option with respect to such Declined NG Development Candidate will terminate, and Sarepta will have no rights to Develop, Manufacture or Commercialize such Declined NG Development Candidate and Summit will have the right to Develop, Manufacture and Commercialize such Declined NG Development Candidate in the Summit Territory and to grant licenses to Third Parties to do the same.

2.4. **Diligence**. Each of the Parties shall use Commercially Reasonable Efforts to execute and to perform, or cause to be performed, the activities assigned to it in the Development Plan and to cooperate with the other Party in carrying out the Development Plan, in each case, in a good scientific manner and in compliance with applicable Law.

2.5. **Records; Reports; Information Sharing**.

2.5.1. **Development Activities**. Each Party will periodically provide to the JSC, but in no event less than once each Calendar Quarter, or more frequently as reasonably requested by the other Party, an update regarding Development activities conducted by or on behalf of such Party.

2.5.2. **Scientific Records**. Each Party will maintain scientific records in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes, which records will fully and properly reflect all work done and results achieved with respect to Licensed Products by such Party.

2.5.3. **Information Exchange and Development Assistance**. Subject to Section 2.2.3(c) and applicable Laws, during the Term, upon the reasonable request of the other Party, each Party shall provide to the other Party, without additional compensation and in a commercially reasonable format, Know-How Controlled by such Party or its Related Parties that is licensed to the other Party under this Agreement (*i.e.*, Know-How included in Sarepta Technology

for Sarepta and Know-How included in Summit Technology for Summit), including copies of (a) all scientific information and data related to the Licensed Products (including all data made, collected or otherwise generated in the conduct of any pre-clinical studies, Clinical Studies or early access/named patient programs for the Licensed Products, as well as CMC information) and (b) protocols and investigator brochures, in each case, that are reasonably necessary for the other Party (or its Related Parties) to perform its obligations or exploit its rights under this Agreement with respect to Licensed Products.

2.5.4. **Personnel**. Each Party may request, through the JSC or the other Party's Collaboration Manager, if the JSC appoints Collaboration Managers, that the other Party reasonably make available for consultation regarding the Development of Licensed Products certain of its employees engaged in Development activities with respect to Licensed Products. The JSC or the Collaboration Managers will reasonably coordinate, upon reasonable notice during normal business hours and at their respective places of employment, consultation between the Parties on the progress of Development of Licensed Products under the Development Plan.

2.5.5. **Confidentiality**. All information exchanged by the Parties under this Section 2.5 will be Confidential Information of the disclosing Party and will be maintained in accordance with Section 9.

2.6. **Third Parties**. The Parties shall be entitled to utilize the services of Third Party contract research and contract manufacturing organizations to perform their respective Development and Commercialization activities under this Agreement; provided that (a) each Party shall require that such Third Party operates in a manner consistent with the terms of this Agreement, (b) each Party shall remain at all times fully liable for its respective responsibilities and (c) each Party shall require that any such Third Party be bound by confidentiality and non-use provisions that are no less stringent than the provisions of Section 9.

3. REGULATORY MATTERS.

3.1. **Regulatory Filings and Interactions**. Each Party shall use Commercially Reasonable Efforts to conduct all regulatory activities relating to Licensed Products in accordance with the then-current Development Plan. Except as otherwise provided in the Development Plan, (a) each Party will own the INDs, the NDAs and related regulatory documents submitted to the applicable Regulatory Authorities in its Territory with respect to Licensed Products and (b) each Party will, as to Licensed Products in its Territory, (i) oversee, monitor and coordinate all regulatory actions, communications and filings with, and submissions to, each Regulatory Authority, (ii) be responsible for interfacing, corresponding and meeting with each Regulatory Authority, (iii) be responsible for maintaining all regulatory filings and (iv) notify the JSC in writing, including a brief description in English of the principal issues raised, of all material communications from Regulatory Authorities within [**], provide the JSC with a summary translation of such material communications in English as soon as reasonably

possible and provide, if appropriate, a full translation of such material communications in English as soon as reasonably possible thereafter. Each Party will provide complete copies of any such original correspondence in their native language to the other Party upon request. Each Party shall provide the other Party with reasonable advance notice of all material, substantive meetings with the Regulatory Authorities in its Territory pertaining to any Licensed Products, or with as much advance notice as practicable under the circumstances. Each Party shall use Commercially Reasonable Efforts, to the extent reasonably practicable, to permit the other Party to have, at the other Party's expense, one (1) mutually acceptable representative of the other Party to attend, solely as an observer, material, substantive meetings with the Regulatory Authorities in the major market countries within such Party's Territory pertaining to any Licensed Product. Each Party shall furnish the other Party with drafts of all copies of such Party's filings and submissions for Regulatory Approval (including draft INDs, NDAs, orphan drug applications and designations) regarding any Licensed Product in such Party's Territory in a timely manner in sufficient time prior to making such filings and submissions to allow the other Party a reasonable opportunity to review and comment thereon and shall consider the other Party's timely comments in good faith. In addition, each Party shall provide the other Party with written notice of (x) all filings and submissions for Regulatory Approval regarding any Licensed Product in such Party's Territory in a timely manner; (y) all Regulatory Approvals obtained or denied; and (z) [**]; provided, however, that in all circumstances, each Party shall inform the other Party of such event prior to public disclosure of such event by such Party.

3.2. **Right of Reference**. Subject to Section 2.2.3(c), each Party hereby grants to the other Party a "**Right of Reference**," as that term is defined in 21 C.F.R. § 314.3(b) (or any successor rule or analogous Law recognized outside of the United States), to, and a right to copy, access and otherwise use, all information and data (including all CMC information as well as data made, collected or otherwise generated in the conduct of any Clinical Studies or early access/named patient programs for the Licensed Products) included in any regulatory filing, Regulatory Approval, drug master file or other regulatory documentation (including orphan drug applications and designations) owned or controlled by such Party or its Related Parties that relates to any Licensed Product, and such Party shall provide a signed statement to this effect, if requested by the other Party, in accordance with 21 C.F.R. § 314.50(g)(3) (or any successor rule or analogous Law outside of the United States).

3.3. **Regulatory Costs; Regulatory Diligence**. Each Party will be responsible for all costs incurred in connection with regulatory activities in support of obtaining any Regulatory Approval for the Licensed Products in its Territory, including the cost of preparing and submitting any NDA with respect to a Licensed Product or interacting with Regulatory Authorities in its Territory (but excluding any such costs incurred in connection with preparing and submitting any IND to applicable Regulatory Authorities, which shall be included in Development Costs and shared by the Parties pursuant to Section 2.2.3). Subject to the Parties' completion of Development sufficient to support such filings, Sarepta shall use Commercially Reasonable Efforts to file NDAs for Licensed Products in the [**] Major European Countries (either directly or through the centralized process with the EMA, and, if such NDA filed for the EMA does not cover the United Kingdom, also in the United Kingdom) and, if Sarepta exercises the Territory Expansion Option, in [**] of the Major Option Countries.

3.4. **Pharmacovigilance**. Within [**] after the Effective Date, or such later time as may be mutually agreed by the Parties, but in any event prior to the commencement of any clinical activities by Sarepta in the Sarepta Territory, the Parties will develop and agree in writing upon a pharmacovigilance agreement ("**Pharmacovigilance Agreement**") that will include safety data exchange procedures governing the coordination of collection, investigation, reporting and exchange of information concerning any adverse experiences and any product quality and product complaints involving adverse experiences, related to Licensed Products, sufficient to enable each Party (and their respective Related Parties, if any) to comply with its legal and regulatory obligations. Unless otherwise agreed by the Parties, the Pharmacovigilance Agreement will [**]. The Pharmacovigilance Agreement will contain terms no less stringent than those required by ICH or other applicable guidelines in order to allow the Parties to meet the applicable regulatory and legal requirements regarding the management of safety data in their respective Territories.

4. COMMERCIALIZATION OF THE LICENSED PRODUCTS

4.1. **Responsibility, Cost and Diligence**.

4.1.1. **Sarepta**. Sarepta shall be solely responsible, at its expense, for all Commercialization activities relating to Licensed Products in the Field in the Sarepta Territory. Sarepta shall use Commercially Reasonable Efforts to Commercialize Licensed Products (a) in each Major European Country, (b) in each country of the CIS, Switzerland, Norway, Iceland and Turkey and (c) if Sarepta exercises the Territory Expansion Option, in [**] of the Major Option Countries, in each case ((a) – (c)), subject to obtaining Regulatory Approval in the applicable country.

4.1.2. **Summit**. Summit shall be solely responsible, at its expense, for all Commercialization activities relating to Licensed Products in the Field in the Summit Territory.

4.1.3. **Joint Commercialization**. In the event that the Parties mutually agree to conduct any joint Commercialization activities regarding a Licensed Product following discussion of such activities by the JSC in accordance with Section 5.3.2(e), the Parties will (a) agree in writing to a written work plan and time table for conducing such activities, (b) agree in writing to management and governance mechanisms for such joint activities, including coordination of such activities through the JSC and (c) negotiate in good faith a budget therefor and an equitable allocation of costs between the Parties.

4.2. **Sarepta's Territory Expansion Option.** Beginning on the Effective Date, Sarepta shall have the option to expand the Sarepta Territory to include the Option Territory (the "**Territory Expansion Option**"). At any time prior to the date that is three (3) months following the first receipt of Regulatory Approval for a Licensed Product in the United

States or in any country in the EU (or from the EMA), Sarepta may exercise the Territory Expansion Option by notifying Summit thereof in writing and paying to Summit [**] (the "**Territory Expansion Option Fee**"). From and after the date on which Sarepta exercises the Territory Expansion Option (the "**Territory Expansion Option Exercise Date**"), the Sarepta Territory shall include the Option Territory. Summit shall not grant to any Third Party any rights with respect to Collaboration Compounds or Licensed Products in the Option Territory prior to the expiration of the Territory Expansion Option.

4.3. Commercialization Plans and Information.

4.3.1. **Sarepta Commercialization Plan**. No less than [**] months in advance of the reasonably expected first Regulatory Approval in the Sarepta Territory with respect to a Licensed Product, and on an annual basis thereafter, Sarepta shall prepare and deliver to the JSC for review a reasonable written plan that summarizes the Commercialization activities to be undertaken with respect to Licensed Products in the Sarepta Territory in the next Calendar Year and, to the extent commercially reasonable, Sarepta's plans to Commercialize Licensed Products in countries in the Sarepta Territory in which Sarepta is not then Commercializing Licensed Products, and the dates by which such activities are targeted to be accomplished (the "**Sarepta Territory Commercialization Plan**"). The Sarepta Territory Commercialization Plan shall subsequently be updated and modified by Sarepta, from time to time at its discretion and no less frequently than once per Calendar Year, based upon, among other things, Sarepta's Commercialization activities with respect to Licensed Products in the Sarepta Territory, a copy of which updated plan Sarepta will provide to the JSC. The Sarepta Territory Commercialization Plan, and each modification thereto, shall be consistent with Sarepta's diligence obligations under Section 4.1.1.

4.3.2. **Summit Commercialization Information**. From time to time as may be reasonably requested by Sarepta's JSC representatives, Summit shall provide to the JSC reasonable summaries of Summit's Commercialization activities with respect to Licensed Products in the Summit Territory and Summit's plans to Commercialize Licensed Products in countries in the Summit Territory, in each case to the extent such information is reasonably relevant and useful for purposes of coordinating the Parties' Commercialization activities and for Sarepta's preparation of the Sarepta Territory Commercialization Plan and amendments thereto.

4.4. Advertising and Promotional Materials.

4.4.1. **Global Branding**. The JSC shall implement (and thereafter modify and update) a global branding strategy (including global positioning, messages, logo, colors and other visual branding elements) jointly developed by the Parties for Licensed Products for use in the Field worldwide (the "**Global Branding Strategy**"). The JSC shall review the Global Branding Strategy at least annually (or more frequently if reasonably requested by either Party) and determine whether to update or modify it.

4.4.2. **Summit**. Summit will be responsible for the creation, preparation, production, reproduction and filing with the applicable Regulatory Authorities, of relevant written sales, promotion and advertising materials relating to Licensed Products ("**Promotional Materials**") for use in the Summit Territory. All such Promotional Material will be (a) compliant with applicable Law and (b) if applicable, consistent in all material respects with the Global Branding Strategy. Summit will submit representative samples of its Promotional Materials developed by it for use in the Summit Territory to the JSC for its review and discussion, at least annually (or more frequently if reasonably requested by Sarepta). Summit shall consider in good faith any timely comments Sarepta may have with respect to such Promotional Materials, but shall have final decision-making authority with respect to such Promotional Materials.

4.4.3. **Sarepta**. Sarepta will be responsible for the creation, preparation, production, reproduction and filing with the applicable Regulatory Authorities, of relevant Promotional Materials for use in the Sarepta Territory. All such Promotional Materials will be (a) compliant with applicable Law, (b) consistent in all material respects with the Sarepta Territory Commercialization Plan and (c) if applicable, consistent in all material respects with the Global Branding Strategy. Sarepta will submit representative samples of its Promotional Materials developed by it for use in the Sarepta Territory to the JSC for its review and discussion at least annually thereafter (or more frequently if reasonably requested by Summit). Sarepta shall consider in good faith any timely comments Summit may have with respect to such Promotional Materials, but shall have final decision-making authority with respect to such Promotional Materials.

4.5. **Reporting Obligations**. Sarepta shall report to the JSC in writing, by no later than each February 28 following the first Regulatory Approval of a Licensed Product in the Field in the Sarepta Territory (for the period ending December 31 of the prior Calendar Year), summarizing Sarepta's Commercialization activities and resources expended for Licensed Products performed to date (or updating such report for activities performed since the last such report was given hereunder, as applicable). In addition, Sarepta shall provide Summit with written notice of the First Commercial Sale of each Licensed Product in the Sarepta Territory within fifteen (15) days after such event; provided, however, that in all circumstances, Sarepta shall inform Summit of such event prior to public disclosure of such event by Sarepta. Each Party shall provide such other information to the JSC as the other Party may reasonably request and shall keep the JSC reasonably informed of such Party's Commercialization activities with respect to Licensed Products.

4.6. **Sales and Distribution**. Each Party and its Related Parties shall be responsible for booking sales and shall warehouse and distribute Licensed Products in its Territory. If a

Party receives any orders for any Licensed Product in the other Party's Territory, then it shall refer such orders to the other Party. Moreover, each Party and its Related Parties shall be solely responsible for handling all returns of Licensed Product sold in its Territory, as well as all aspects of Licensed Product order processing, invoicing and collection, distribution, inventory and receivables of Licensed Products sold in its Territory.

4.7. **Recalls, Market Withdrawals or Corrective Actions**. In the event that any Regulatory Authority issues or requests a recall or takes a similar action in connection with the Licensed Products in a Territory, or in the event either Party determines that an event, incident or circumstance has occurred that may result in the need for a recall, market withdrawal or stock recovery in its own Territory, the Party notified of such recall or similar action, or the Party that desires such recall or similar action, shall within twenty-four (24) hours and in all cases prior to the execution of such recall, market withdrawal or stock recovery advise the other Party thereof by telephone, facsimile or e-mail (except in the case of a government mandated recall, when such Party may not provide such advance notice but shall notify the other Party as soon as possible). Each Party, in consultation with the other Party, shall decide whether to conduct a recall in its own Territory and the manner in which any such recall shall be conducted (except in the case of a government mandated recall, when such Party may act without such advance consultation but shall notify the other Party as soon as possible). Each Party shall be responsible for the execution of any such recall in its own Territory, and in each such case the other Party shall take such actions as reasonably requested by the executing Party in connection therewith and otherwise reasonably cooperate in all such efforts. Except as otherwise provided in a Supply Agreement, each Party shall bear the expense of any such recall in its own Territory, provided that Summit shall reimburse Sarepta for the expense of any such recall in the Sarepta Territory to the extent such recall is the result of a Manufacturing defect in Licensed Product supplied by (or on behalf of) Summit to Sarepta. In addition, each Party will make available all of its pertinent records that may be reasonably requested by the other Party in order to effect a recall in the other Party's Territory.

4.8. **Commercial Expenses**. Except where otherwise specifically set forth in this Agreement, each Party shall bear all costs and expenses incurred in connection with its Commercialization of Licensed Products in its Territory.

4.9. **Ex-Territory Sales; Export Monitoring**.

4.9.1. **Ex-Territory Sales**. Subject to applicable Law, neither Party shall engage in any advertising or promotional activities relating to any Licensed Product directed primarily to customers or other buyers or users of such Licensed Product located outside its Territory or accept orders for Licensed Products from or sell Licensed Products into such other Party's Territory for its own account, and if a Party receives any order for Licensed Products in the other Party's Territory, then it shall refer such orders to the other Party.

4.9.2. **Export Monitoring**. Each Party and its Related Parties will use Commercially Reasonable Efforts to monitor and prevent exports of Licensed Products from its own Territory for Commercialization in the other Party's Territory using methods commonly used in the industry for such purpose, and shall promptly inform the other Party of any such exports of Licensed Products from its Territory, and the actions taken to prevent such exports. Each Party agrees to take reasonable actions requested in writing by the other Party that are consistent with Law to prevent exports of Licensed Products from its Territory for Commercialization in the other Party's Territory.

5. COLLABORATION MANAGEMENT

5.1. **Joint Steering Committee**. The Parties hereby establish a joint steering committee (the "**JSC**") to facilitate the Collaboration as follows:

5.1.1. **Composition of the Joint Steering Committee**. The Collaboration shall be conducted under the oversight of the JSC, which shall be comprised of [**] representatives of each Party. Each Party shall appoint its respective representatives to the JSC and may substitute any of its representatives, in its sole discretion, effective upon notice to the other Party of such change. Additional representatives or consultants may from time to time, by mutual consent of the Parties, be invited to attend JSC meetings, subject to such representatives and consultants undertaking confidentiality obligations, whether in a written agreement or by operation of law, no less stringent than the provisions of Section 9.

5.1.2. **JSC Chairperson**. The JSC chairperson shall rotate every [**] between a JSC representative of Summit and a JSC representative of Sarepta. The initial JSC chairperson shall be a representative of [**]. The JSC chairperson's responsibilities shall include (a) scheduling meetings at least once per Calendar Quarter, but more frequently if the JSC determines it necessary; (b) setting agendas for meetings with solicited input from other members; (c) coordinating the delivery of draft minutes to the JSC for review and final approval; and (d) conducting meetings, including ensuring that objectives for each meeting are set and achieved.

5.2. **Appointment of Subcommittees, Project Teams and Collaboration Managers**. The JSC shall be empowered to create such subcommittees and project teams as it may deem appropriate or necessary. Each such subcommittee and project team shall report to the JSC, which shall have the authority to approve or reject recommendations or actions proposed thereby subject to the terms of this Agreement. The JSC may direct each Party to designate a Collaboration manager to serve as a primary point of contact for the other Party under the Collaboration (the "**Collaboration Manager**"). Each Party may change its Collaboration Manager at any time in its sole discretion with written notice to the other Party.

5.3. **Meetings**. The JSC shall meet in accordance with a schedule established by mutual written agreement of the Parties, but no less frequently than once per Calendar Quarter during the Term, with the location for such meetings alternating between Summit and Sarepta facilities (or such other locations as are mutually agreed by the Parties). Alternatively, the JSC may meet by means of teleconference, videoconference or other similar communications equipment, but at least two (2) meetings per Calendar Year shall be conducted in person. Each Party shall bear its own expenses relating to attendance at such meetings by its representatives.

5.3.1. **JSC Development Responsibilities**. The JSC shall have the following responsibilities with respect to the Development of Licensed Products pursuant to the Collaboration:

(a) Reviewing, approving or declining to approve the initial Development Plan as set forth in Section 2.2, and preparing, reviewing, approving or declining to approve proposed amendments to, the Development Plan, in each case, in accordance with Section 2.2.2;

(b) monitoring, planning and coordinating the Development of Licensed Products and regularly assessing the progress of the Parties in their conduct of the Development Plan against the timelines and budgets contained therein, reviewing relevant data;

(c) reviewing updates regarding the Development of Licensed Products provided by the Parties pursuant to Section 2.5.1 (or otherwise);

(d) approving or declining to approve Additional Studies in accordance with Section 2.2.3(c);

(e) establishing procedures for maintaining and recording Development Costs and, in accordance with Section 2.2.3(b), assisting the Parties in conducting a reconciliation of Development Costs in each Calendar Quarter;

(f) reviewing and discussing material communications received from Regulatory Authorities in accordance with Section 3.1;

(g) assisting the Parties to conduct a reconciliation of Development Costs for the subject Calendar Quarter within ten (10) days after receipt of all such supporting information pursuant to Section 2.2.3(b);

(h) overseeing the manufacturing relationship between the Parties with respect to the Manufacture and supply of Licensed Products for Development activities pursuant to Section 6.1 and the Clinical Supply Agreement;

(i) designating or declining to designate Next Generation Collaboration Compounds as a Development Candidate in accordance with Section 2.3;

(j) serving as a forum for the Parties' discussions of intellectual property issues, including potential opportunities identified by the Parties for licensing Third Party intellectual property; and

(k) performing such other activities as the Parties agree in writing shall be the responsibility of the JSC.

5.3.2. **JSC Commercialization Responsibilities**. The JSC shall have the following responsibilities with respect to the Commercialization of Licensed Products pursuant to the Collaboration, to the extent permissible under applicable Laws:

(a) implementing, modifying and updating the Global Branding Strategy pursuant to Section 4.4.1;

(b) reviewing and discussing the Sarepta Territory Commercialization Plan, including updates thereto provided by Sarepta pursuant to Section 4.3.1 and Summit's Commercialization information in its Territory provided by Summit pursuant to Section 4.3.2;

(c) reviewing and discussing Promotional Material for use in each Party's Territory in accordance with Sections 4.4.2 and 4.4.3;

(d) providing a forum for the Parties to discuss the Commercialization of Licensed Products in the Field worldwide, including coordination regarding Licensed Product positioning and messaging, key opinion leader relationship management, medical affairs and marketing and selling materials;

(e) providing a forum for the Parties to discuss collaborating on commercial activities that can be leveraged for both Parties' respective Territories agreed to by the Parties in accordance with Section 4.1.3 and how the Parties would share the costs of such mutually agreed joint Commercialization activities;

(f) overseeing the manufacturing relationship between the Parties with respect to the Manufacture of Licensed Products for Commercialization activities pursuant to Section 6.1 and the Commercial Supply Agreement;

(g) reviewing and discussing the Product Trademark(s) proposed for use by Sarepta and its Related Parties throughout the Sarepta Territory pursuant to Section 12.8.2; and

(h) performing such other activities as the Parties agree in writing shall be the responsibility of the JSC.

5.4. Decision-Making.

5.4.1. **Voting; Consensus**. With respect to decisions of the JSC, the representatives of each Party shall have collectively one vote on behalf of such Party. For each meeting of the JSC, [**]. Action on any matter may be taken at a meeting by teleconference, videoconference or by written agreement. The JSC shall attempt to resolve any and all disputes before it for decision by consensus.

5.4.2. **Escalation to CEOs**. If the JSC is unable to reach consensus with respect to a dispute for a period in excess of [**], then the dispute shall be submitted to the Chief Executive Officers of Summit and Sarepta, or their designees (any such designee to be a senior member of the designating Chief Executive Officer's management team) for resolution.

5.4.3. **Tie-Breaking Authority**. If such dispute cannot be resolved for a period in excess of [**] following escalation, then:

(a) *Sarepta Matters*. Subject to clause (c) below, the Chief Executive Officer of Sarepta or his or her designee shall have the deciding vote on any matter involving the Commercialization of Licensed Products in the Field in the Sarepta Territory;

(b) *Summit Matters.* Subject to clause (c) below, the Chief Executive Officer of Summit or his or her designee shall have the deciding vote on any matter involving [**]; and

(c) *Matters Reserved for Consensus*. Neither Party shall have the deciding vote on any of the following matters:

(i) [**];

(ii) [**];

(iii) [**];

(iv) [**];

(v) [**];

(vi) [**];

(vii) [**]; or

(viii) [**].

5.4.4. **No Authority to Amend**. Notwithstanding anything to the contrary set forth herein, the JSC shall not have the authority to modify the terms of this Agreement or take any action to expand or narrow the responsibilities of the JSC.

5.5. **Third Party Partners**. If, at any time during the Term, a Party enters into an agreement with one or more Third Party(ies) (a "**Third Party Partner**") (each such agreement, a "**Third Party Collaboration Agreement**", and the Party entering such Third Party Collaboration Agreement the "**Contracting Party**") to Develop or Commercialize a Licensed Product in the Field in the Contracting Party's Territory, then the Contracting Party shall ensure that such agreement is consistent with the terms and conditions of this Agreement. Without limiting the foregoing, the Contracting Party shall use reasonable efforts to negotiate terms in the Third Party Agreement regarding (a) intellectual property rights necessary to permit the Contracting Party to license or sublicense to the other Party, in accordance with the terms of this Agreement, any Patent Rights and Know-How developed in the course of activities pursuant to the Third Party Collaboration Agreement related to the Licensed Products that are the subject of this Agreement, if any, and (b) providing the other Party with reciprocal information, rights of reference and other rights and benefits with respect to regulatory matters in the Third Party Partner's Territory as are provided to the other Party in Section 3.2. The Contracting Party shall promptly provide the other Party with a copy of any fully executed Third Party Collaboration Agreement, which may be redacted to remove terms and conditions that are not necessary to monitor compliance with this Section 5.5 and such Third Party Collaboration Agreement will be the Contracting Party's Confidential Information for the purposes of Section 9. In addition, if the Third Party Collaboration Agreement grants the Third Party Partner a sublicense under the Sarepta Technology or Summit Technology, as applicable, then the Contracting Party shall ensure that such Third Party Collaboration agreement complies with Section 7.1.2 or Section 7.2.2, as applicable. If the Contracting Party becomes aware of a material breach of the terms of such Third Party Collaboration Agreement by a Third Party Partner compliance with which is necessary for the Contracting Party's compliance with the terms of this Agreement, then the Contracting Party shall promptly notify the other Party of the particulars of the same and use Commercially Reasonable Efforts to cause the Third Party Partner to comply with all the terms of the Third Party Collaboration Agreement necessary for the Contracting Party's compliance with the terms of this Agreement. Notwithstanding any Third Party Collaboration Agreement, the Contracting Party shall remain primarily liable to the other Party for the performance of the Contracting Party's obligations under, and the Contracting Party's compliance with all terms and conditions of, this Agreement with respect to the Contracting Party's Territory.

6. MANUFACTURE AND SUPPLY OF THE LICENSED PRODUCT

6.1. **Supply Obligations**. From and after the Effective Date, but subject to a right of Summit to terminate such supply obligations on [**] prior written notice to Sarepta, subject to the Supply Agreements once entered into pursuant to Section 6.2, Summit will use Commercially Reasonable Efforts, either itself or through Third Parties, to Manufacture API Bulk Drug Substance, Finished Drug Product and placebo meeting all applicable

product specifications as filed in the IPMD and other applicable regulatory filings, in accordance with applicable current Good Manufacturing Practices and equivalent Laws outside the United States ("**cGMP**"), and supply to Sarepta API Bulk Drug Substance, Finished Drug Product and placebo (as applicable) in quantities that are reasonably sufficient for the conduct of Development and Commercialization by Sarepta with respect to the Sarepta Territory under the Development Plan and the Sarepta Territory Commercialization Plan, respectively. For any API Bulk Drug Substance or Finished Drug Product supplied by Summit to Sarepta pursuant to this Section 6.1 for purposes of Commercialization in the Sarepta Territory, Sarepta shall pay to Summit an amount equal to [**] of Summit's Cost of Goods for such API Bulk Drug Substance or Finished Drug Product (as applicable), payable within forty-five (45) days after receipt of an invoice therefor. Except with respect to any Additional Studies as to which a Non-Proposing Party has not opted in in accordance with Section 2.2.3(c), [**] of Summit's Cost of Goods incurred in Manufacturing API Bulk Drug Substance, Finished Drug Product and placebo for purposes of Development worldwide shall be Development Costs borne by the Parties in accordance with Section 2.2.3. With respect to any Additional Study as to which the Non-Proposing Party has not opted into in accordance with Section 2.2.3(c), [**] of Summit's Cost of Goods incurred in Manufacturing API Bulk Drug Substance, Finished Drug Product and placebo for purposes of conducting such Additional Study shall be borne solely by the Proposing Party, and, if such Party is Sarepta, then Sarepta shall pay such amounts to Summit within [**] days after receipt of an invoice therefor.

6.2. **Supply Agreements**. Within [**] after the Effective Date, the Parties will negotiate in good faith and enter into (a) a supply agreement for clinical supply of Licensed Products and placebo (the "**Clinical Supply Agreement**") and a related quality agreement, if the Development Plan anticipates a need for Summit to supply Sarepta in order to enable Sarepta to carry out Development activities allocated to it in the Development Plan and (b) a supply agreement for commercial supply of Licensed Products (the "**Commercial Supply Agreement**" and together with the Clinical Supply Agreement, the "**Supply Agreements**") and a related quality agreement, which Supply Agreements will each be consistent with the terms set forth in Schedule 6.2 and the terms set forth in Section 6.1 with respect to clinical supply of Licensed Product. Notwithstanding anything to the contrary set forth herein, when the Parties enter into the Supply Agreements, the terms of such Supply Agreements shall supersede the terms set forth in Section 6.1.

6.3. **Arbitration for Failure to Agree**. If the Parties cannot reach agreement and enter into any Supply Agreement (or related quality agreement) within the applicable period set forth in Section 6.2, then the final terms and conditions of such Supply Agreement (or related quality agreement) will be determined through binding arbitration as follows:

6.3.1. **Manufacturing Arbitration Drafts**. Each Party will (a) prepare a draft of such Supply Agreement (or related quality agreement) (which will be consistent with the applicable terms set forth on Schedule 6.2 the terms set forth in Section 6.1 with respect to clinical supply of Licensed Product for Additional Studies) to be used in such arbitration proceeding (each, a "**Manufacturing Arbitration Draft**") and (b) submit its Manufacturing Arbitration Draft to the other Party. Within fifteen (15) days of such

submissions, the Parties will meet to determine whether or not they agree to enter into either Party's Manufacturing Arbitration Draft or a modified version thereof as such Supply Agreement (or related quality agreement).

6.3.2. **Notice; Experts**. If the Parties are unable to agree within the fifteen (15) day period set forth in Section 6.3.1, then either Party may send the other Party written notice that it wishes to determine the final terms and conditions of such Supply Agreement using a Neutral Expert. Within thirty (30) days of a Party's receipt of such notice, the Parties shall jointly appoint a neutral Third Party who is an expert with at least fifteen (15) years of experience in the area of manufacturing and supply (the "**Neutral Expert**") within ten (10) business days.

6.3.3. **Resolution by Arbitration**. Within three (3) business days of such meeting, each Party may submit an opposition statement of no more than five (5) pages in length to the Neutral Expert. Neither Party will be allowed to conduct any discovery. Neither Party may have any communications (either written or oral) with the Neutral Expert other than for the sole purpose of engaging the Neutral Expert or as expressly permitted in this Section 6.3.3. The Neutral Expert may consult in writing with either Party regarding the submissions made by either Party; provided that both Parties receive such request for consultation and are provided with an opportunity to respond. In evaluating each Party's written submissions, the Neutral Expert shall, within ten (10) business days of receipt of the written opposition statement, select one of the Parties' Manufacturing Arbitration Drafts within thirty (30) days following the receipt of the latter of such Manufacturing Arbitration Drafts and select the draft that it determines to contain the most fair, balanced and customary terms (in addition to reflecting the applicable terms set forth on Schedule 6.2 and the applicable terms set forth in Section 6.1 with respect to clinical supply of Licensed Product for Additional Studies); provided that, the Neutral Expert shall not select, and Summit shall not be required to execute or perform under, any Supply Agreement that would require Summit to undertake obligations that cannot be satisfied through the use of Commercially Reasonable Efforts. Such decision shall be final, binding and conclusive upon both Parties and their Affiliates, and such Manufacturing Arbitration Draft will be the applicable Supply Agreement (or related quality agreement), and the Parties will execute the same.

6.3.4. **Responsibility for Costs**. The fees of the Neutral Expert will be borne by the Party whose Manufacturing Arbitration Draft is not selected by the arbitral tribunal.

6.4. **Establishment of Second Source and Back-Up Sources**. If the Parties have entered into a Supply Agreement, and Sarepta notifies Summit that it desires that a second source be established for the concurrent Manufacture and supply of a Licensed Product for clinical or commercial use (a "**Second Source**") or that a back-up supplier be established for the contingent Manufacture and supply of a Licensed Product for clinical or

commercial use (a "**Back-Up Source**"), then Summit shall reasonably assist Sarepta in establishing a Second Source or Back-Up Source on a timeline agreed by the Parties and in accordance with the terms of this Section 6.4. Notwithstanding the foregoing, Sarepta may not provide such notification to Summit requesting that a Second Source or Back-Up Source be established unless and until [**]. A Second Source or Back-Up Source established by Sarepta may also serve as a Second Source or Back-Up Source for Summit. In addition, if Summit desires to establish a Second Source or a Back-Up Source (as applicable) for a Licensed Product for clinical or commercial use, then Summit shall notify Sarepta in advance of Summit's commencement of material negotiations with one or more Third Party manufacturers relating to the establishment of a Second Source or Back-Up Source (as applicable) for the Manufacture and supply of such Licensed Product.

6.5. **Transfer of Manufacturing Know-How**. During the Term, upon Sarepta's request, Summit shall transfer to Sarepta and to the applicable Second Source and Back-Up Sources described in Section 6.4 all Summit Know-How then Controlled by Summit that is reasonably necessary or useful to enable the Manufacture of each Licensed Product for clinical or commercial use and not previously transferred to Sarepta under this Agreement by providing copies or samples of relevant documentation, materials and other embodiments of such Know-How, and by making available its qualified technical personnel on a reasonable basis to consult with Sarepta, the Second Source and Back-Up Sources, as applicable, with respect to such Know-How. Each such Know-How transfer requested by Sarepta for itself, for the Second Source or for a Sarepta-selected Back-Up Source ("**Technology Transfer**") shall be commenced within a commercially reasonable timeframe following Sarepta's request and conducted pursuant to an agreed technology transfer plan to be developed by the Parties (with input from the Second Source or Back-Up Sources, as applicable) for the purpose of ensuring the complete and timely transfer of such Know-How in a manner that is consistent with then-current internal technology transfer corporate standards (or equivalent policy) of Sarepta or the Second Source or Back-Up Sources, as applicable (to the extent a copy of such standards or equivalent policy has been provided to Summit). The cost of any such Technology Transfer shall be borne by the Parties as if such cost were Development Costs and Summit's personnel costs therefor shall be computed using the FTE Rate (pro-rated for partial FTE usage). Summit's Out-of-Pocket Costs incurred in the course of such Technology Transfers shall also be borne by the Parties as if such costs were Development Costs, provided that such Out-of-Pocket Costs are incurred in accordance with the agreed technology transfer plan.

7. LICENSES; EXCLUSIVITY

7.1. **License Grants to Sarepta**.

7.1.1. **Development, Manufacturing and Commercialization Licenses**.

(a) Development and Manufacturing License. Subject to the terms and conditions of this Agreement, Summit hereby grants Sarepta a non-transferable (except as provided in Section 14.2), sublicensable (subject to Section 7.1.2), royalty-free, co-exclusive (with Summit) license in

the Sarepta Territory and non-exclusive license in the Summit Territory under the Summit Technology to Develop and Manufacture (or have Manufactured) Licensed Products in the Field.

(b) Commercialization License. Subject to the terms and conditions of this Agreement, Summit hereby grants Sarepta a non-transferable (except as provided in Section 14.2), sublicensable (subject to Section 7.1.2) exclusive (even as to Summit) license under the Summit Technology to Commercialize Licensed Products in the Field in the Sarepta Territory. Such license shall be royalty-bearing for the Royalty Term applicable to each Licensed Product in each country in the Sarepta Territory, and, after the Royalty Term applicable to such Licensed Product in such country, shall convert to a fully-paid, irrevocable, perpetual license to Commercialize such Licensed Product in the Field in such country.

7.1.2. Sublicensing Terms.

(a) Permitted Sublicensees. Subject to the requirements of this Section 7.1.2 and the provisions of any Summit In-License, Sarepta shall have the right to sublicense any of its rights under Section 7.1.1 to any of its Affiliates or to any Third Party (which sublicensed rights may be further sublicensable through multiple tiers) without the prior consent of Summit, [**].

(b) Sublicense Agreements. Each sublicense granted by Sarepta pursuant to this Section 7.1.2 shall be subject and subordinate to the terms and conditions of this Agreement and shall contain terms and conditions consistent with those in this Agreement. Sarepta shall promptly provide Summit with a copy of the fully executed sublicense agreement covering any sublicense granted to a Third Party hereunder (which copy may be redacted to remove terms and conditions that are not necessary to monitor compliance with this Section 7.1.2), and shall provide Summit with notice identifying any Affiliate Sublicensee. Each sublicense agreement (whether with an Affiliate or a Third Party) shall contain the following provisions: [**].

(c) Continuation of Sublicenses upon Termination of this Agreement. If the licenses granted to Sarepta under Section 7.1 are terminated by Summit prior to expiration of the Term pursuant to Section 13.2.2, then, at the request of any Sublicensee who is not then in breach of its sublicense agreement, Summit will enter into, without any assistance by Sarepta, a direct license agreement with such Sublicensee under the Summit Technology that is sublicensed to such Sublicensee with substantially the same scope as set forth in such sublicense agreement between Sarepta and such Sublicensee; provided, however, that [**].

(d) Sublicensee Breach. If Sarepta becomes aware of a material breach of the terms of any sublicense by any Sarepta Sublicensee that it is necessary for Sarepta's compliance with the terms of this Agreement, then Sarepta shall promptly notify Summit of the particulars of the same and shall use Commercially Reasonable Efforts to cause the Sublicensee to comply with all of the terms of the sublicense agreement necessary for Sarepta's compliance with the terms of this Agreement. In the event that (i) the Sublicensee has failed to cure a material breach within sixty (60) days after notice of such breach and (ii) such material breach also constitutes a material breach of this Agreement, Sarepta shall terminate the sublicense agreement at the request of Summit. Notwithstanding any sublicense, Sarepta shall remain primarily liable to Summit for the performance of all of Sarepta's obligations under, and Sarepta's compliance with all terms and conditions of, this Agreement.

7.2. License Grants to Summit.

7.2.1. Development, Manufacturing and Commercialization Licenses.

(a) Development and Manufacturing License. Subject to the terms and conditions of this Agreement, Sarepta hereby grants Summit a non-transferable (except as provided in Section 14.2), sublicensable (subject to Section 7.2.2), non-exclusive, royalty-free license under the Sarepta Technology to Develop and Manufacture (or have Manufactured) Licensed Products in the Field worldwide.

(b) Commercialization License. Subject to the terms and conditions of this Agreement, Sarepta hereby grants Summit a non-transferable (except as provided in Section 14.2), sublicensable (subject to Section 7.2.2), non-exclusive, royalty-free license under the Sarepta Technology to Commercialize Licensed Products in the Field in the Summit Territory.

7.2.2. Sublicensing Terms.

(a) Permitted Sublicensees. Subject to the requirements of this Section 7.2.2, Summit shall have the right to sublicense any of its rights under Section 7.2.1 to any of its Affiliates or to any Third Party (which sublicensed rights may be further sublicensable through multiple tiers) without the prior consent of Sarepta.

(b) Sublicense Agreements. Each sublicense granted by Summit pursuant to this Section 7.2.2 shall be subject and subordinate to the terms and conditions of this Agreement and shall contain terms and conditions consistent with those in this Agreement. Summit shall promptly provide Sarepta with a copy of the fully executed sublicense agreement covering any sublicense granted hereunder (which copy may be

redacted to remove terms and conditions that are not necessary to monitor compliance with this Section 7.2.2), and such sublicense agreement shall contain the following provisions: [**].

(c) Sublicensee Breach. If Summit becomes aware of a material breach of the terms of any sublicense by any Summit Sublicensee that it is necessary for Summit's compliance with the terms of this Agreement, then Summit shall promptly notify Sarepta of the particulars of the same and use Commercially Reasonable Efforts to cause the Sublicensee to comply with all of the terms of the sublicense agreement necessary for Summit's compliance with the terms of this Agreement. In the event that (i) the Sublicensee has failed to cure a material breach within sixty (60) days after notice of such breach and (ii) such material breach also constitutes a material breach of this Agreement, Summit shall terminate the sublicense agreement at the request of Sarepta. Notwithstanding any sublicense, Summit shall remain primarily liable to Sarepta for the performance of all of Summit's obligations under, and Summit's compliance with all terms and conditions of, this Agreement.

7.3. In-Licenses.

7.3.1. Compliance with In-Licenses. All licenses and other rights granted to Sarepta under this Section 7 are subject to the rights and obligations of Summit under the Summit In-Licenses. All licenses and other rights granted to Summit under this Section 7 are subject to the rights and obligations of Sarepta under the Sarepta In-Licenses. As of the Effective Date there are no Sarepta In-Licenses. Each Party shall comply with all applicable terms and conditions of the In-Licenses, and shall perform and take such actions as may be required to allow the Party that is party to such In-License to comply with its obligations thereunder, including obligations relating to sublicensing, patent matters, confidentiality, reporting, audit rights, indemnification and diligence; provided that, in all cases, a Party that is not party to an In-License shall not have any obligation to comply, or to perform such actions as may be required to allow the other Party to comply, with any terms and conditions of such In-Licenses that have been redacted from the copies of such In-Licenses disclosed to such Party. Without limiting the foregoing, each Party shall prepare and deliver to the other Party any additional reports required under the applicable In-Licenses, in each case sufficiently in advance to enable the Party that is party to such In-License to comply with its obligations under the applicable In-Licenses. Each Party agrees, upon the other Party's request, to provide the other Party with copies of any In-Licenses to which it is a party. Confidential Information of the providing Party or its counterparty may be redacted from such copies, except to the extent that such information is required in order to enable the other Party to comply with its obligations to the providing Party under this Agreement with respect to such In-License or in order to enable the providing Party to ascertain compliance with the provisions of this Agreement.

7.3.2. **Payments Under In-Licenses**. Subject to Section 8.4.6, [**]. If either Party breaches its payment obligation to a licensor under a an In-License and the other Party determines, in its sole discretion, to pay any such Third Party License Payment directly to such licensor in order to cure the first Party's default and avoid losing the rights sublicensed to the other Party under such In-License, then the other Party may (but will not be obligated to) make such payments directly to such licensor. In such event either [**].

7.3.3. **Breach or Termination of In-Licenses**. In the event that (a) a Party receives notice of an alleged breach by such Party under an In-License to which it is a party or (b) a Party intends to terminate an In-License that it is a party to, then, in either case ((a) or (b)), such Party will promptly, but in no event less than ten (10) days thereafter, provide written notice thereof to the other Party.

7.3.4. **Freedom to Obtain New In-Licenses.** For the avoidance of doubt, each Party shall be free to enter into new In-Licenses following the Effective Date in order to avoid infringement or misappropriation of any Third Party's Patent Rights, Know-How or other intellectual property rights in such Party's Territory or obtain access to Patent Rights, Know-How or other intellectual property that may be reasonably necessary or useful to the Development, Manufacture or Commercialization of Licensed Products in such Party's Territory.

7.4. **Bankruptcy**. All rights and licenses granted to either Party pursuant to any section of this Agreement, including pursuant to Section 7, are licenses of rights to "intellectual property" (as defined in Section 101(35A) of title 11 of the United States Code (the "**Bankruptcy Code**")). Each Party shall retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against either Party or any of its Affiliates (collectively, the "**Bankrupt Party**") under the Bankruptcy Code or analogous provisions of applicable Law outside the United States, the other Party (the "**Non-Bankrupt Party**"), as a licensee under the Bankrupt Party's intellectual property, shall be entitled to a complete duplicate of (or complete access to, as appropriate) such intellectual property and all embodiments of such intellectual property, which, if not already in the Non-Bankrupt Party's possession, shall be promptly delivered to it upon the Non-Bankrupt Party's request therefor.

7.5. **No Other Rights**. Except as otherwise expressly provided in this Agreement, under no circumstances shall a Party, as a result of this Agreement, obtain any ownership interest or other right in any Know-How, Patent Rights or other intellectual property rights of the other Party, including items owned, controlled or developed by the other Party, or provided by the other Party to the receiving Party at any time pursuant to this Agreement.

7.6. **Exclusivity**. During the Term and, if this Agreement is terminated by Sarepta pursuant to Section 13.2.1 or by Summit pursuant to Section 13.2.2 or 13.2.4, for one (1) year after such termination of this Agreement, other than as part of the Collaboration, then neither Sarepta nor any of its Affiliates shall, itself or with or through any Third Party, without

the prior written consent of Summit, engage in any Commercialization of any Competing Product. Likewise, subject to Section 2.3, during the Term, and if this Agreement is terminated by Sarepta pursuant to Section 13.2.2, then for one (1) year after such termination of this Agreement, other than as part of the Collaboration, neither Summit nor any of its Affiliates shall, itself or with or through any Third Party, without the prior written consent of Sarepta, engage in any Commercialization of any Competing Product; [**].

7.7. Competing Product Acquisitions.

7.7.1. **Options**. If, during the term of the exclusivity covenant in Section 7.6, a Party (the "**Acquired Party**") or any of its Affiliates acquires or is acquired by a Third Party (whether such acquisition occurs by way of a purchase of assets, merger, consolidation or similar transaction), and where such Third Party is, at such time, actively Commercializing a Competing Product, unless the Parties agree otherwise in writing, then the Acquired Party, or its applicable Affiliate, will (with respect to the applicable Competing Product), at its option and no later than ninety (90) days following the date of consummation of the relevant merger, consolidation or acquisition, notify the other Party in writing of its determination to either:

(a) divest, or cause the relevant Affiliate to divest, whether by license or otherwise, its interest in the Competing Product, to the extent necessary to be in compliance with Section 7.6;

(b) terminate the Commercialization of the Competing Product; or

(c) if the Acquired Party is Sarepta, terminate this Agreement pursuant to Section 13.2.1.

7.7.2. **Divestiture or Termination**. If the Acquired Party notifies the other Party in writing that it or its relevant Affiliate intends to divest such Competing Product or terminate either this Agreement (if the Acquired Party is Sarepta) or the Commercialization of the Competing Product as provided in Section 7.7.1, then the Acquired Party or its relevant Affiliate will effect the consummation of such divestiture within twelve (12) months or effect such termination within six (6) months, subject to compliance with applicable Law (as applicable), after the consummation of the relevant merger, consolidation or acquisition contemplated in Section 7.7.1, and will confirm to the other Party in writing when such divestiture or termination has been completed. The Acquired Party will keep Summit reasonably informed of its efforts and progress in effecting such divestiture or termination until it is completed.

8. CERTAIN FINANCIAL TERMS

8.1. **Upfront Fee**. In consideration for the rights, licenses and options granted by Summit to Sarepta under this Agreement, within ten (10) days after the Effective Date, Sarepta shall pay Summit a non-refundable, non-creditable initial payment of Forty Million U.S. Dollars ($40,000,000).

8.2. Development Milestone Fees.

8.2.1. **First Licensed Product**. Subject to the terms and conditions of this Agreement, Sarepta shall make the non-refundable, non-creditable milestone payments to Summit set forth in TABLE 8.2.1 below, each payable once, no later than forty-five (45) days after the earliest date on which the corresponding milestone event has first been achieved with respect to the first Licensed Product to achieve such milestone event.

TABLE 8.2.1: First Licensed Product

Milestone Event	Milestone Payment
(i) Administration of the first dose to the last patient to receive a first dose in the PhaseOut DMD Clinical Study*	$ 22,000,000
(ii) [**]	[**]
(iii) [**]	[**]
(iv) [**]	[**]

* Patients enrolled as part of the safety arm cohort to the PhaseOut DMD Clinical Study shall not be considered to be patients in the PhaseOut DMD Clinical Study for the purposes of establishing whether the last patient has been dosed in such PhaseOut DMD Clinical Study.

(a) Timing of First Milestone Payment. If the milestone event in row (i) is achieved prior to April 1, 2017, then the milestone payment set forth in row (i) shall be due on the later of April 1, 2017 or forty-five (45) days after the date on which such event was achieved.

(b) [**] Determination. If a [**] was not considered a [**], but later is determined to be a [**], then the milestone event set forth in row (ii) shall be deemed to have occurred on the date that such determination is made.

(c) Deemed [**]. If the milestone event set forth in row (ii) of TABLE 8.2.1 has not yet occurred, and [**] is [**] for a [**], then, upon such event, a [**] shall also be deemed to [**] with respect to such Licensed Product for purposes of this Section 8.2.1.

(d) Deemed [**]. If the milestone event set forth in row (iii) of TABLE 8.2.1 has not yet occurred, and a Licensed Product [**], then, upon such event, [**] shall also be deemed to [**] for such Licensed Product for purposes of this Section 8.2.1.

(e) Milestone Triggering. For the avoidance of doubt, each milestone amount set forth in this Section 8.2.1 shall be payable no more than once, but all three milestones need not be achieved by the same Licensed Product in order to trigger Sarepta's payment obligations. For example, if a [**] with respect to a Licensed Product, then Sarepta shall

pay to Summit [**]. If the Parties then [**] with respect to such Licensed Product [**] with respect to such Licensed Product is [**], and [**] is then [**] with respect to a [**] Licensed Product, then Sarepta shall be under no obligation to pay Summit for such [**]. However, if the milestone event set forth in row (iii) of TABLE 8.2.1 has not yet occurred and [**] in the Sarepta Territory with respect to such [**] Licensed Product, Sarepta shall pay to Summit [**].

8.2.2. **Subsequent Licensed Products**. Subject to the terms and conditions of this Agreement, after the first Regulatory Approval has been obtained for a Licensed Product, then, with respect to each additional Licensed Product being Developed by the Parties, on a Licensed Product-by-Licensed Product basis Sarepta shall make the non-refundable, non-creditable milestone payments to Summit set forth in TABLE 8.2.2, each payable once per additional Licensed Product, below no later than forty-five (45) days after the earliest date on which the corresponding milestone event has first been achieved with respect to each such Licensed Product.

TABLE 8.2.2: Subsequent Licensed Products

Milestone Event	Milestone Payment
(i) [**]	[**]
(ii) [**]	[**]
(iii) [**]	[**]
(iv) [**]	[**]
(v) [**]	[**]

(a) Cumulative Payments. If the date on which the first Regulatory Approval in the Sarepta Territory is obtained for the first Licensed Product is later in time than the date(s) on which one or more of the milestone events set forth in TABLE 8.2.2 above have been achieved with respect to one or more other Licensed Product(s), then Sarepta shall pay all milestone payments associated with such milestone event(s) and Licensed Product(s) within forty-five (45) days of the date on which such first Regulatory Approval is obtained.

(b) Deemed [**]. If a [**] was not considered a [**], but later is determined to be a [**], then the milestone event set forth in row (iii) of TABLE 8.2.2 shall be deemed to have occurred on the date that such determination is made.

(c) Deemed [**]. If the milestone event set forth in row (i) of TABLE 8.2.2 has not yet occurred, and a [**] with respect to a Licensed Product, then, upon such event, [**] shall also be deemed to [**] for such Licensed Product for purposes of this Section 8.2.2.

(d) Deemed [**]. If the milestone event set forth in row (ii) of TABLE 8.2.2 has not yet occurred, and a [**] is [**] with respect to a Licensed Product, then, upon such event a [**] shall also be deemed to [**] with respect to such Licensed Product for purposes of this Section 8.2.2.

(e) Deemed [**]. If either of the milestone events set forth in row (ii) or (iii) of TABLE 8.2.2 has not yet occurred, and [**] for a Licensed Product is [**], then, upon such event, a [**] shall also be deemed to [**] with respect to such Licensed Product for purposes of this Section 8.2.2.

(f) Deemed [**]. If the milestone event set forth in row (iv) of TABLE 8.2.2 has not yet occurred, and a Licensed Product [**], then, upon such event, [**] for a Licensed Product shall also be deemed to [**] for purposes of this Section 8.2.2.

8.2.3. **Option Territory Milestone Fees**. In addition to the milestone fees set forth in Sections 8.2.1 and 8.2.2, if Sarepta exercises the Territory Expansion Option by paying the Territory Expansion Option Fee pursuant to Section 4.2, then Sarepta shall also make the non-refundable, non-creditable milestone payments to Summit set forth in TABLE 8.2.3 below, no later than forty-five (45) days after the earliest date on which the corresponding milestone event has first been achieved with respect to the first Licensed Product to achieve such milestone event.

TABLE 8.2.3: Licensed Product in a Major Option Country

Milestone Event	Milestone Payment
(i) [**]	[**]
(ii) [**]	[**]
(iii) [**]	[**]

(a) Deemed [**]. If the milestone event set forth in row (i) of TABLE 8.2.3 has not yet occurred, and a Licensed Product [**] in any Major Option Country, then, upon such event, [**] shall also be deemed to [**] for such Licensed Product in each other Major Option Country for purposes of this Section 8.2.3.

(b) Milestone Triggering. The milestone payments set forth in row (i) and (ii) of TABLE 8.2.3 shall be payable only once, regardless of how many times such milestone is achieved. The milestone payment in row (iii) of TABLE 8.2.3 shall be payable up to (but not more than) [**] times. However, all three milestones in TABLE 8.2.3 need not be achieved by the same Licensed Product in order to trigger Sarepta's payment obligations. For example, [**] to a Licensed Product in [**], then Sarepta shall pay to Summit [**]. If the Parties then cease Development activities with respect to such Licensed Product before a [**] is received for such Licensed Product in [**], and [**] is then [**] in [**] with respect to a [**] Licensed Product, then Sarepta shall not be obligated to pay Summit for the acceptance of such [**]. However, if the milestone event set forth in row (ii) of TABLE 8.2.3 has not yet occurred and [**] for such [**] Licensed Product in [**], then Sarepta shall pay to Summit [**].

8.2.4. **Notification of Milestone Events**. Sarepta shall provide Summit with written notice of the achievement by Sarepta or any of its Related Parties of any milestone event set forth in Sections 8.2.1, 8.2.2 or 8.2.3 within five (5) days after such event; provided, however, that Sarepta shall inform Summit of such event at least three (3) days prior to any public disclosure of such event by Sarepta.

8.3. **Sales Milestone Fees.**

8.3.1. **Sarepta Territory Sales by Sarepta or its Related Parties**. Subject to the terms and conditions of this Agreement, on a Licensed Product-by-Licensed Product basis, Sarepta shall make the non-refundable, non-creditable milestone payments to Summit set forth in TABLE 8.3.1 below, each payable once, no later than forty-five (45) days after the earliest date on which the corresponding milestone event has first been achieved by Sarepta or its Related Parties with respect to such Licensed Product in the Sarepta Territory.

TABLE 8.3.1: Sarepta Territory Sales Milestone Fees

Milestone Event	Milestone Payment
(i) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]
(ii) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]
(iii) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]
(iv) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]
(v) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]

The milestone payments set forth in TABLE 8.3.1 above shall each be payable only once for each Licensed Product, upon the first achievement of the applicable Net Sales threshold with respect to such Licensed Product in a given Calendar Year. If more than one of such milestone events first occurs based on sales of a Licensed Product in the same Calendar Year, then all of such milestone payments shall be paid for such Calendar Year.

8.3.2. **Option Territory Sales by Sarepta or its Related Parties**. Subject to the terms and conditions of this Agreement, in addition to the sales milestone fees set forth in Section 8.3.1, on a Licensed Product-by-Licensed Product basis, Sarepta shall also make the non-refundable, non-creditable milestone payments to Summit set forth in TABLE 8.3.2 below, each payable once, no later than forty-five (45) days after the earliest date on which the corresponding milestone event has first been achieved by Sarepta or its Related Parties with respect to such Licensed Product in the Option Territory.

TABLE 8.3.2: Option Territory Sales Milestone Fees

Milestone Event	Milestone Payment
(i) Calendar Year Net Sales of the Licensed Product in the Option Territory equal to or greater than [**]	[**]
(ii) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]
(iii) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]
(iv) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]
(v) Calendar Year Net Sales of the Licensed Product in the Sarepta Territory equal to or greater than [**]	[**]

The milestone payments set forth in TABLE 8.3.2 above shall each be payable only once for each Licensed Product, upon the first achievement of the applicable Net Sales threshold with respect to such Licensed Product in a given Calendar Year. If more than one of such milestone events first occurs based on sales of a Licensed Product in the same Calendar Year, then all of such milestone payments shall be paid for such Calendar Year.

8.4. Royalties.

8.4.1. **Royalty Rates**. Subject to the terms and conditions of this Agreement, on a Licensed Product-by-Licensed Product basis, Sarepta shall pay to Summit royalties on the aggregate Calendar Year Net Sales of such Licensed Product in the Sarepta Territory as set forth in TABLE 8.4.1 below:

TABLE 8.4.1: Royalty Rates

Aggregate Annual Net Sales of such Licensed Product in the Sarepta Territory	Royalty Rate
First [**]	[**]
Portion above [**] and equal to or below [**]	[**]
Portion above [**]	[**]

8.4.2. **Tiered Payments**. Royalties payable pursuant to this Section 8.3.2 shall be paid at the rate applicable to the portion of Net Sales within each of the Net Sales levels during the applicable Calendar Year for the relevant Licensed Product. For example, if, during a Calendar Year, aggregate Net Sales of a Licensed Product were equal to [**] then the royalties payable by Sarepta would be calculated by adding (a) the royalties with respect to the first [**] at the [**] percentage of [**] equal to [**] and (b) the royalties with respect to the next [**] at the [**] percentage of [**] equal to [**] for a total royalty of [**].

8.4.3. **Royalty Term**. The term of Sarepta's royalty obligations to Summit pursuant to this Section 8.4 shall apply on a country-by-country and Licensed Product-by-Licensed Product basis during the applicable Royalty Term in such country for such Licensed Product. Following the expiration of the applicable Royalty Term in such country for such Licensed Product (but not following an earlier termination of this Agreement), the licenses granted to Sarepta pursuant to

Section 7.1 with respect to such Licensed Product in such country shall be fully-paid, irrevocable, perpetual and royalty-free, on a Licensed Product-by-Licensed Product and country-by-country basis.

8.4.4. **[**]**. Subject to Section 8.4.7, on a country-by-country and Licensed Product-by-Licensed Product basis, the royalties to be paid by Sarepta to Summit pursuant to this Section 8.4 for such Licensed Product shall be reduced to [**] of the amounts otherwise payable pursuant to Section 8.4.1 with respect to Net Sales in such country of the Sarepta Territory for such Licensed Product if both [**].

8.4.5. **Royalty Adjustments for Generic Products**. Subject to Section 8.4.7, if, during a given Calendar Quarter when a Licensed Product is being Commercialized by or on behalf of Sarepta in a particular country in the Sarepta Territory, there is Generic Competition in such country with respect to a Licensed Product, then, subject to Section 8.4.6, the royalties payable on the Net Sales of such Licensed Product in such country shall thereafter be reduced to [**] of the amounts otherwise payable pursuant to Section 8.4.1 with respect to such Licensed Product in such country for such Calendar Quarter for so long as such Generic Competition remains. Notwithstanding the foregoing, if there is Generic Competition for a period of [**] with respect to a Licensed Product, then thereafter the royalty adjustment in this Section 8.4.5 will continue to apply for the remainder of the Royalty Term applicable to such Licensed Product in such country, regardless of whether Generic Competition continues to exist.

8.4.6. **Royalty Anti-Stacking**. Subject to Section 8.4.7, if Sarepta (a) determines in good faith that, in order to avoid infringement of any Third Party's Patent Rights not licensed to Sarepta hereunder, it is reasonably necessary to obtain a license after the Effective Date from a Third Party under Patent Rights owned or licensable by such Third Party Covering such Licensed Product (but excluding any such Patent Rights (i) to the extent Covering a use of such Licensed Product that is not indicated in a Regulatory Approval for such Licensed Product in such country or (ii) owned or licensable by a contract manufacturing organization engaged by Sarepta) in order to Manufacture or Commercialize a Licensed Product in the Field in a country in the Sarepta Territory and to pay a royalty under such license (including in connection with the settlement of a patent infringement claim) or (b) becomes subject to a final court or other binding order or ruling requiring the payment of a royalty or damages to a Third Party patent holder in respect of the Manufacture or Commercialization of a Licensed Product in the Field in a country in the Sarepta Territory, then, on a country-by-country basis, in each case, ((a) and (b)), the amount of Sarepta's royalty payments under Section 8.4.1 with respect to Net Sales for such Licensed Product in such country in any Calendar Quarter shall be reduced by [**] of the payments actually paid by Sarepta to such Third Party in consideration for such license that are reasonably and appropriately allocable to such Licensed Product in the Field in such country during such Calendar Quarter.

8.4.7. **Royalty Floor**. Notwithstanding the foregoing provisions of this Section 8.4, in no event during the applicable Royalty Term for a Licensed Product in a country of the Sarepta Territory shall the royalties payable to Summit hereunder for such Licensed Product in such country for any Calendar Quarter be reduced pursuant to Sections 8.4.4, 8.4.5 and 8.4.6 to less than [**] of the royalties payable pursuant to Section 8.4.1 as to such Licensed Product in such country for such Calendar Quarter.

8.4.8. **Reports; Payment of Royalty**. During the Term, following the First Commercial Sale of a Licensed Product in the Sarepta Territory, Sarepta shall furnish to Summit a written report within forty-five (45) days after the end of each Calendar Quarter showing, on a Licensed Product-by-Licensed Product and country-by-country basis, the gross sales of each Licensed Product in each country of the Sarepta Territory, deductions from gross sales (itemized by deduction category) for each Licensed Product for each country of the Sarepta Territory included in the calculation of Net Sales, the Net Sales in each country of the Sarepta Territory of Licensed Product during the reporting period and the royalties payable under this Agreement. Quarterly reports shall be due no later than the forty-fifth (45th) day following the end of each Calendar Quarter. In addition Sarepta shall prepare and deliver to Summit any additional reports as required under the Summit In-Licenses. Royalties shown to have accrued by each royalty report shall be due and payable on the date such royalty report is due. Sarepta and its Related Parties shall keep complete and accurate records in sufficient detail to enable the royalties and other payments payable hereunder and by Summit to Third Parties under the Summit In-Licenses to be determined.

8.5. **Audits**.

8.5.1. **Records; Inspections**. Each Party shall keep complete and accurate records of the items underlying Development Costs, Declined NG Candidate Development Costs, Net Sales, Cost of Goods, royalties, milestones, other license fees and other payments under this Agreement. Upon the written request of a Party and not more than once in each Calendar Year, the other Party and its Related Parties shall permit an independent certified public accounting firm of internationally-recognized standing selected by the requesting Party and reasonably acceptable to the other Party, at the requesting Party's expense except as set forth below, to have access during normal business hours to such of the records of the other Party as may be reasonably necessary to verify the accuracy of the payments and reports hereunder for any year ending not more than three (3) years prior to the date of such request for the sole purpose of verifying the basis and accuracy of payments made under this Agreement.

8.5.2. **Discrepancies**. If such accounting firm identifies a discrepancy made during such period, then the appropriate Party shall pay the other Party the amount of the discrepancy (together with, in the case of any underpayments, late-payment interest in accordance with Section 8.7) within fifteen (15) days after the date the requesting Party delivers to the other Party such accounting firm's written report so concluding, or as otherwise agreed by the Parties in writing. The fees charged by such accounting firm shall be paid by the requesting Party, unless such discrepancy represents an underpayment by the other Party of at least [**] of the total amounts due hereunder in the audited period, in which case such fees shall be paid by the other Party.

8.5.3. **Compliance with In-Licenses**. Each Party shall comply with all applicable audit requirements in the In-Licenses and shall include in each sublicense granted by it pursuant to this Agreement a provision requiring the Sublicensee to make reports to the Party that is party to such In-License, to keep and maintain records of sales made pursuant to such sublicense and to grant access to such records by the independent accountant of the Party that is party to such In-License to the same extent required of a Party under this Agreement.

8.5.4. **Audit Term**. Unless an audit for such year has been commenced prior to and is ongoing upon the third (3rd) anniversary of the end of such year, the calculation of payments payable with respect to such year shall be binding and conclusive upon both Parties, and each Party and its Related Parties shall be released from any further liability or accountability with respect to such royalties or expense reimbursement for such year.

8.5.5. **Confidential Treatment**. Each Party shall treat all financial information subject to review under this Section 8.5 or under any sublicense agreement in accordance with the confidentiality and non-use provisions of this Agreement, and shall cause its accounting firm to enter into a written confidentiality agreement with the other Party or its Related Parties obligating it to retain all such information in confidence pursuant to such confidentiality agreement, which terms shall be no less stringent than the provisions of Section 9.

8.6. **Payment Exchange Rate**. Each payment to be made to Summit under this Agreement shall be made in such currency and to such bank account in the United Kingdom as may be designated in writing by Summit from time to time. All payments to be made under this Agreement to Sarepta shall be made in United States dollars and shall be paid by bank wire transfer in immediately available funds to such bank account in the United States as may be designated in writing by Sarepta from time to time. If, in a given Calendar Quarter, either Party is required to convert between currencies in order to make a payment in accordance with this Section 8.6, then such Party shall make such conversion using the average rate of exchange for such Calendar Quarter utilized by such Party in its worldwide accounting system and calculated in accordance with GAAP.

8.7. **Late Payments**. Any amount owed by a Party to the other Party under this Agreement that is not paid on or before the date such payment is due shall bear interest at a rate per

annum equal to the lesser of (a) the then-current one (1) month London Inter-Bank Offering Rate for US Dollars, as quoted on the British Banker's Association's website currently located at www.bba.org.uk (or such other source as may be mutually agreed by the Parties) plus [**] per annum or (b) the highest rate permitted by Law, calculated on the number of days such payments are paid after such payments are due and compounded monthly.

8.8. **Blocked Payments**. If, by reason of applicable Laws in any jurisdiction in a Party's Territory, it becomes impossible or illegal for a Party to transfer milestone payments, royalties or other payments under this Agreement to the other Party, then the payor shall promptly notify the payee. During any such period described above, the payor shall deposit such payments in local currency in the relevant jurisdiction to the credit of the payee in a recognized banking institution designated by the payee or, if none is designated by the payee within a period of ninety (90) days, in a recognized banking institution selected by the payor and identified in a written notice given to the payee.

8.9. **Taxes**. If a timely and appropriately completed and executed Internal Revenue Service Form W-9 is provided by the receiving Party to the paying Party, then the Parties acknowledge and agree that no United States tax withholding shall be applied with respect to the payments due under this Agreement. Each Party shall use reasonable efforts to minimize tax withholding on payments made to the other Party. Notwithstanding such efforts, if such Party concludes that tax withholdings under the Laws of any country are required with respect to payments to the other Party, then such Party shall first notify the other Party and provide such Party with twenty (20) days to determine whether there are actions such receiving Party can undertake to avoid such withholding. During this notice period, the paying Party shall refrain from making such payment until the receiving Party instructs the paying Party that (a) the receiving Party intends to take actions (satisfactory to both Parties) that will obviate the need for such withholding, in which case the paying Party shall make such payment only after it is instructed to do so by the receiving Party or (b) the paying Party should make such payment and withhold the required amount and pay it to the appropriate Governmental Authority. In such case, the withholding Party shall promptly provide the other Party with copies of receipts or other evidence reasonably required and sufficient to allow the other Party to document such tax withholdings adequately for purposes of claiming foreign tax credits and similar benefits. The Parties will cooperate reasonably in completing and filing documents required under the provisions of any applicable tax laws or under any other applicable Law, in connection with the making of any required tax payment or withholding payment, or in connection with any claim to a refund of or credit for any such payment. The Parties will cooperate to minimize such taxes in accordance with applicable Laws, including using reasonable efforts to access the benefits of any applicable treaties. Notwithstanding the foregoing, if, as a result of (y) the assignment of this Agreement by Sarepta to an Affiliate or a Third Party outside of the United States or (z) the exercise by Sarepta of its rights under this Agreement through an Affiliate or Third Party outside of the United States (or the direct exercise of such rights by an Affiliate of Sarepta outside of the United States), foreign withholding tax in excess of the foreign withholding tax amount that would have been payable in the absence of such assignment or exercise of rights becomes payable with respect to amounts due to Summit hereunder, then such amount

due to Summit will be increased so that the amount actually paid to Summit equals the amount that would have been payable to Summit in the absence of such excess withholding (after withholding of the excess withholding tax and any additional withholding tax on such increased amount). However, if a similar assignment or exercise of rights described in clauses (y) or (z) of the preceding sentence by Summit results in foreign withholding tax in excess of the foreign withholding tax amount that would have been payable in the absence of such assignment or exercise of rights, then any amount due to Summit will not be increased for such excess withholding and, subject to the terms of this Agreement, the required amount will be withheld and submitted to the appropriate Governmental Authority.

9. CONFIDENTIALITY AND PUBLICATION

9.1. Nondisclosure Obligation.

9.1.1. **Non-Disclosure and Non-Use; Exceptions**. During the Term and for a period of five (5) years thereafter, all Confidential Information disclosed by one Party to the other Party hereunder shall be maintained in confidence by the receiving Party and shall not be disclosed to a Third Party or used for any purpose except as set forth herein without the prior written consent of the disclosing Party, except to the extent that such Confidential Information:

(a) is known by the receiving Party at the time of its receipt, and not through a prior disclosure by the disclosing Party, as documented by the receiving Party's business records;

(b) is known to the public before its receipt from the disclosing Party, or thereafter becomes generally known to the public through no breach of this Agreement by the receiving Party;

(c) is subsequently disclosed to the receiving Party by a Third Party who is not known by the receiving Party to be under an obligation of confidentiality to the disclosing Party; or

(d) is developed by the receiving Party independently of Confidential Information received from the disclosing Party, as documented by the receiving Party's business records.

9.1.2. **Permitted Disclosures**. Notwithstanding the obligations of confidentiality and non-use set forth above, a receiving Party may provide Confidential Information disclosed to it, and disclose the existence and terms of this Agreement, as may be reasonably required in order to perform its obligations and to exploit its rights under this Agreement, and specifically to (a) Related Parties, and their employees, directors, agents, consultants, advisors or other Third Parties for the performance of its obligations hereunder (or for such entities to determine their interest in performing such activities) in accordance with this Agreement in each case, who are under an obligation of confidentiality with respect to such information that is no less stringent than

the terms of this Section 9; (b) governmental or other Regulatory Authorities in order to obtain patents or perform its obligations or exploit its rights under this Agreement; provided that such Confidential Information shall be disclosed only to the extent reasonably necessary to do so, (c) the extent required by Law, including by the rules or regulations of the United States Securities and Exchange Commission or similar regulatory agency in a country other than the United States or of any stock exchange or listing entity, (d) any bona fide actual or prospective acquirers, underwriters, investors, lenders or other financing sources and any *bona fide* actual or prospective licensee, sublicensees, collaborators or strategic partners and to consultants and advisors of such Party, in each case, who are under an obligation or confidentiality with respect to such information that is no less stringent than the terms of this Section 9 and (e) to Third Parties to the extent a Party is required to do so pursuant to the terms of an Existing Summit In-License. If a Party is required by Law to disclose Confidential Information that is subject to the non-disclosure provisions of this Section 9, then such Party shall promptly inform the other Party of the disclosure that is being sought in order to provide the other Party an opportunity to challenge or limit the disclosure obligations. Confidential Information that is required to be disclosed by Law shall remain otherwise subject to the confidentiality and non-use provisions of this Section 9. If either Party concludes that a copy of this Agreement must be filed with the United States Securities and Exchange Commission or similar regulatory agency in a country other than the United States, then such Party will provide the other Party with a copy of this Agreement showing any provisions hereof as to which the Party proposes to request confidential treatment, will provide the other Party with an opportunity to comment on any such proposed redactions and to suggest additional redactions and will take such Party's reasonable comments into consideration before filing the Agreement.

9.2. Publication and Publicity.

9.2.1. **Publication**. Sarepta and Summit each acknowledge the other Party's interest in publishing certain key results of the Collaboration. Each Party also recognizes the mutual interest in obtaining valid patent protection and in protecting trade secret information. Consequently, except for disclosures permitted pursuant to Section 9.1 and Section 9.2.3, either Party wishing to make a publication or public presentation that contains the Confidential Information of the other Party shall deliver to the other Party a copy of the proposed written publication or presentation a reasonable period of time prior to submission for publication or presentation. The reviewing Party shall have the right (a) to propose modifications to the publication or presentation for patent reasons, trade secret reasons or business reasons, and the publishing Party will remove all Confidential Information of the other Party if requested by the reviewing Party and (b) to request a reasonable delay in publication or presentation in order to protect patentable information. If the reviewing Party requests a delay, then the publishing Party shall delay submission or presentation for a period of ninety (90) days (or such shorter period as may be

mutually agreed by the Parties) to enable the non-publishing Party to file patent applications protecting such Party's rights in such information in accordance with Section 12. With respect to any proposed publications or disclosures by investigators or academic or non-profit collaborators, such materials shall be subject to review under this Section 9.2.1 to the extent that Sarepta or Summit, as the case may be, has the right and ability (after using Commercially Reasonable Efforts to obtain such right and ability) to do so.

9.2.2. **Publicity; Use of Names**. Except as set forth in Section 9.1 and Section 9.2.3, no Party shall use the name, trademark, trade name or logo of the other Party or its employees in any publicity, news release or disclosure relating to this Agreement or its subject matter without the prior express written permission of the other Party, except as may be required by Law or expressly permitted by the terms hereof.

9.2.3. **Press Release**. Following the execution of this Agreement, the Parties shall issue a joint press release in the form set forth in Schedule 9.2.3. After such initial press release, neither Party shall issue press releases or make public disclosures relating to this Agreement or the terms hereof, including relating to the Development, Manufacture or Commercialization of Licensed Products, unless (a) the information in such release or disclosure has been previously publicly disclosed and is materially true and correct at the time of the subsequent disclosure or (b) the Party making the disclosure provides the other Party with a draft of such proposed disclosure at least two (2) business days (or, to the extent timely disclosure of a material event is required by Law or stock exchange or stock market rules, such period of time sufficiently in advance of the disclosure so that the other Party will have the opportunity to comment upon the disclosure) prior to making any such disclosure, for the other Party's review and comment, and the disclosing Party shall consider in good faith any timely comments provided by the other Party.

10. REPRESENTATIONS, WARRANTIES AND COVENANTS

10.1. **Mutual Representations and Warranties**. Each Party represents and warrants to the other Party that as of the Effective Date:

10.1.1. It is duly organized and validly existing under the laws of its jurisdiction of incorporation or formation, and has full corporate or other power and authority to enter into this Agreement, and to carry out the provisions hereof.

10.1.2. It is duly authorized to execute and deliver this Agreement, and to perform its obligations hereunder, and the person or persons executing this Agreement on its behalf has been duly authorized to do so by all requisite corporate action.

10.1.3. This Agreement is legally binding upon it and enforceable in accordance with its terms. The execution, delivery and performance of this Agreement by it does not conflict with any agreement, instrument or understanding, oral or written, to which it is a party and by which it may be bound, or with its charter or by-laws.

10.1.4. It has not granted any right to any Third Party that would conflict with the rights granted to the other Party hereunder.

10.1.5. Neither Party nor any of its Affiliates has been debarred or is subject to debarment pursuant to Section 306 of the United States Federal Food, Drug, and Cosmetic Act, as amended, or that is the subject of a conviction described in such section.

10.2. Representations and Warranties of Summit. Summit represents and warrants to Sarepta that as of the Effective Date:

10.2.1. **Ownership or Control**. Summit is the sole and exclusive owner of all Summit Patent Rights set forth on Schedule 1.89 as of the Effective Date. All of such Summit Technology solely and exclusively owned by Summit is free and clear of claims, liens, charges or encumbrances that are inconsistent with the rights granted to Sarepta under this Agreement.

10.2.2. **Authority**. Summit has sufficient legal or beneficial title and ownership of, or sufficient license rights under, the Summit Technology to grant the licenses to such Summit Technology granted to Sarepta pursuant to this Agreement.

10.2.3. **Summit Patent Rights**. (a) Schedule 1.89 collectively sets forth a complete and accurate list of the Summit Patent Rights, (b) to Summit's knowledge, each issued Summit Patent Right remains in full force and effect and (c) Summit or its Affiliates have paid all filing and renewal fees required to be paid on or before the Effective Date with respect to such Summit Patent Rights.

10.2.4. **Completeness of Schedules**. Other than the Summit Patent Rights set forth on Schedule 1.89, Summit does not Control any Patent Rights that Cover any Collaboration Compound or Licensed Product.

10.2.5. **Infringement**. To Summit's knowledge, the Development, Manufacture and Commercialization of Benzoxazole Collaboration Compounds or Benzoxazole Licensed Products will not infringe the intellectual property rights of any Third Party. Except for Know-How and Patent Rights Controlled by contract manufacturers engaged by Summit as of the Effective Date, Summit Controls all Know-How, and to its knowledge Controls all Patent Rights, in each case, used in the Manufacture, Development and Commercialization of the Licensed Products. There is (a) no claim, action or proceeding pending, (b) no written communication (other than general letters received by Summit regarding assays not specific to any Collaboration Compound) or (c) to Summit's knowledge, no threatened claim, action or proceeding, in each case ((a), (b) and (c)) alleging that the Development, Manufacture or Commercialization of any Collaboration Compound or Licensed Product, the activities of Summit or

any of its Affiliates with respect to any such Collaboration Compound or Licensed Product, or the practice or use of the Summit Patent Rights or Summit Know-How, infringes or misappropriates any Patent Rights or other intellectual property of any Third Party.

10.2.6. **Validity**. To Summit's knowledge, the Summit Patent Rights in the Sarepta Territory existing as of the Effective Date, are, or, upon issuance, will be, valid and enforceable patents and no Third Party has challenged or threatened to challenge the scope, validity or enforceability of any such Summit Patent Right (including, by way of example, through opposition or the institution or written threat of institution of interference, nullity or similar invalidity proceedings before the United States Patent and Trademark Office or any analogous foreign Governmental Authority).

10.2.7. **Diligent Prosecution and Maintenance**. Summit and its Affiliates have complied with all applicable Laws, including any duties of candor to applicable patent offices, in connection with the filing, prosecution and maintenance of the Summit Patent Rights existing as of the Effective Date.

10.2.8. **Existing Summit In-Licenses**. Subject to the "Non-Commercial Use" (as defined in the University of Oxford Option Agreement) rights retained by Oxford under the University of Oxford Option Agreement, and the rights retained by Oxford to the extent Summit does not exercise the options therein, none of the Existing Summit In-Licenses (or any agreement to which Summit is a party) contain provisions that conflict with the exclusive rights and licenses granted to Sarepta hereunder or cause Summit to cease to Control any Summit Technology.

10.2.9. **No Defaults under Existing Summit In-Licenses**. To Summit's knowledge, neither Summit nor its Affiliates are in breach or default under any Existing Summit In-License, and neither Summit nor its Affiliates have received any written notice of breach or default with respect to any Existing Summit In-License.

10.2.10. **Invention Assignments**. Summit has obtained from all inventors of Summit Technology owned by Summit as of the Effective Date valid and enforceable agreements assigning to Summit each such inventor's entire right, title and interest in and to all such Summit Technology.

10.2.11. **Absence of Litigation**. There is no (a) claim, demand, suit, proceeding, arbitration, inquiry, investigation or other legal action of any nature, civil, criminal, regulatory or otherwise, pending or, to Summit's knowledge, threatened against Summit or any of its Affiliates or (b) judgment or settlement against or owed by Summit or any of its Affiliates, in each case, in connection with the Summit Technology existing as of the Effective Date.

10.3. **Representations and Warranties of Sarepta**. Sarepta represents and warrants to Summit as of the Effective Date that is not a party to any agreement with a Third Party under which it Controls Know-How or Patent Rights that are reasonably necessary or useful to Develop or Commercialize Licensed Products in the Field in the Summit Territory or that would require Summit to make any payment in connection with Summit's or its Related Parties' Development or Commercialization of Licensed Products in the Field in the Summit Territory.

10.4. **Warranty Disclaimer**. EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS AGREEMENT, NEITHER PARTY MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, TO THE OTHER PARTY WITH RESPECT TO ANY TECHNOLOGY, LICENSED PRODUCT, GOODS, SERVICES, RIGHTS OR OTHER SUBJECT MATTER OF THIS AGREEMENT AND HEREBY DISCLAIMS ALL IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE AND NON-INFRINGEMENT WITH RESPECT TO ANY AND ALL OF THE FOREGOING. EACH PARTY HEREBY DISCLAIMS ANY REPRESENTATION OR WARRANTY THAT THE DEVELOPMENT, MANUFACTURE OR COMMERCIALIZATION OF THE LICENSED PRODUCTS PURSUANT TO THIS AGREEMENT WILL BE SUCCESSFUL OR THAT ANY PARTICULAR SALES LEVEL WITH RESPECT TO THE LICENSED PRODUCTS WILL BE ACHIEVED.

10.5. **Mutual Covenants**.

10.5.1. **Non-Contravention**. During the Term, neither Party, nor its Related Parties, will grant any right to any Third Party that would conflict with the rights granted to the other Party hereunder. Each Party will comply with each In-License to which it is a Party and will not materially breach or otherwise take any action that would permit the licensor thereunder to terminate such In-License without the prior written consent of the other Party if such termination would adversely affect the rights of the other Party hereunder.

10.5.2. **Compliance with Laws**. Each Party and its Related Parties shall conduct the Collaboration and the Development, Manufacture and Commercialization of the Licensed Products in accordance with all Laws, including applicable governmental regulations concerning good laboratory practices, good clinical practices and good manufacturing practices.

10.5.3. **Debarment**. Neither Party nor any of its Affiliates will use in any capacity, in connection with the Collaboration or the performance of its obligations under this Agreement, any Person that has been debarred pursuant to Section 306 of the United States Federal Food, Drug, and Cosmetic Act, as amended, or that is the subject of a conviction described in such section. Each Party agrees to inform the other Party in writing immediately if it or any Person that is performing activities in the Collaboration or under this Agreement, is debarred or is subject to debarment or is the subject of a conviction described in Section 306, or if any action, suit, claim, investigation or legal or administrative

proceeding is pending or, to the best of the notifying Party's knowledge, is threatened, relating to the debarment or conviction of the notifying Party or any Person used in any capacity by such Party or any of its Affiliates in connection with the Collaboration or the performance of its other obligations under this Agreement.

10.6. **Summit Spending Commitment**. So long as Development activities with respect to the Benzoxazole Licensed Product have not ceased, Summit shall spend a total of at least [**] in the performance of its activities under the Development Plan prior to the end of 2019.

11. INDEMNIFICATION; LIMITATION OF LIABILITY; INSURANCE

11.1. **General Indemnification by Sarepta**. Sarepta shall indemnify, hold harmless, and defend Summit, its Related Parties, and their respective directors, officers, employees and agents ("**Summit Indemnitees**") from and against any and all Third Party claims, suits, losses, liabilities, damages, costs, fees and expenses (including reasonable attorneys' fees and litigation expenses) (collectively, "**Losses**") arising out of or resulting from, directly or indirectly, (a) any breach of, or inaccuracy in, any representation or warranty made by Sarepta in this Agreement, or any breach or violation of any covenant or agreement of Sarepta in or in the performance of this Agreement or (b) the negligence or willful misconduct by or of Sarepta or its Related Parties, and their respective directors, officers, employees and agents in the performance of Sarepta's obligations under this Agreement. Sarepta shall have no obligation to indemnify the Summit Indemnitees to the extent that the Losses arise out of or result from, directly or indirectly, any breach of, or inaccuracy in, any representation or warranty made by Summit in this Agreement, or any breach or violation of any covenant or agreement of Summit in or in the performance of this Agreement, or the negligence or willful misconduct by or on behalf of any of the Summit Indemnitees, or matters for which Summit is obligated to indemnify Sarepta under Section 11.2 or Section 11.3.

11.2. **General Indemnification by Summit**. Summit shall indemnify, hold harmless, and defend Sarepta, its Related Parties and their respective directors, officers, employees and agents ("**Sarepta Indemnitees**") from and against any and all Losses arising out of or resulting from, directly or indirectly, (a) any breach of, or inaccuracy in, any representation or warranty made by Summit in this Agreement, or any breach or violation of any covenant or agreement of Summit in or in the performance of this Agreement or (b) the negligence or willful misconduct by or of Summit or its Related Parties, and their respective directors, officers, employees and agents in the performance of Summit's obligations under this Agreement. Summit shall have no obligation to indemnify the Sarepta Indemnitees to the extent that the Losses arise out of or result from, directly or indirectly, any breach of, or inaccuracy in, any representation or warranty made by Sarepta in this Agreement, or any breach or violation of any covenant or agreement of Sarepta in or in the performance of this Agreement, or the negligence or willful misconduct by or on behalf of any of the Sarepta Indemnitees, or matters for which Sarepta is obligated to indemnify Summit under Section 11.1 or Section 11.3.

11.3. **Product Liability**. Any Losses arising out of Third Party product liability claims arising from manufacturing defects in Licensed Products Manufactured by Summit shall be borne by Summit. Any other Losses arising out of Third Party product liability claims arising from the Development or Commercialization of Licensed Products shall be (a) borne by Sarepta, to the extent such Losses were incurred with respect to the Development or Commercialization of the Licensed Products in or for the Sarepta Territory by or on behalf of Sarepta and its Related Parties and (b) be borne by Summit, to the extent such Losses were incurred with respect to Development or Commercialization of the Licensed Products in or for the Summit Territory by or on behalf of Summit and its Related Parties. The Party bearing such Losses in accordance with this Section 11.3 shall indemnify, hold harmless and defend the other Party and its Related Parties and their respective directors, officers, employees and agents from and against such Losses.

11.4. **Indemnification Procedure**. In the event of any indemnified claim against any Sarepta Indemnitee or Summit Indemnitee (individually, an "**Indemnitee**"), the indemnified Party shall promptly notify the other Party in writing of the claim and the indemnifying Party shall manage and control, at its sole expense, the defense of the claim and its settlement; provided, however, that the indemnifying Party may not settle the claim without the indemnified Party's prior written consent (not to be unreasonably withheld), if such settlement materially adversely impacts the indemnified Party's rights or obligations. The Indemnitee shall cooperate with the indemnifying Party and may, at its option and expense, be represented in any such action or proceeding. The indemnifying Party shall not be liable for any settlements, litigation costs or expenses incurred by any Indemnitee without the indemnifying Party's written authorization. Notwithstanding the foregoing, if the indemnifying Party believes that any of the exceptions to its obligation of indemnification of the Indemnitees set forth in Sections 11.1, 11.2 or 11.3 may apply, then the indemnifying Party shall promptly notify the Indemnitees, who shall then have the right to be represented in any such action or proceeding by separate counsel at their expense; provided that the indemnifying Party shall be responsible for payment of such expenses if the Indemnitees are ultimately determined to be entitled to indemnification from the indemnifying Party for the matters to which the indemnifying Party notified the Indemnitees that such exception(s) may apply.

11.5. **Limitation of Liability**. NEITHER PARTY WILL BE LIABLE FOR SPECIAL, INDIRECT, INCIDENTAL, EXEMPLARY, CONSEQUENTIAL OR PUNITIVE DAMAGES ARISING OUT OF THIS AGREEMENT OR THE EXERCISE OF ITS RIGHTS HEREUNDER, INCLUDING LOST PROFITS ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES, EXCEPT AS A RESULT OF A PARTY'S WILLFUL MISCONDUCT OR A BREACH OF THE CONFIDENTIALITY AND NON-USE OBLIGATIONS IN SECTION 9. NOTHING IN THIS SECTION 11.5 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY.

11.6. **Insurance**. Each Party shall maintain insurance during the Term and for a period of at least two (2) years after the last commercial sale of any Licensed Product under this

Agreement, with a reputable, solvent insurer in an amount appropriate for its business and products of the type that are the subject of this Agreement, and for its obligations under this Agreement. Specifically, each Party shall maintain product liability insurance and clinical trial liability insurance with limits of at least [**] per occurrence and in annual aggregate. Upon request, each Party shall provide the other Party with evidence of the existence and maintenance of such insurance coverage.

12. INTELLECTUAL PROPERTY OWNERSHIP, PROTECTION AND RELATED MATTERS

12.1. **Ownership**. Except as otherwise expressly set forth in this Agreement, Summit retains all of its rights, title and interests in and to the Summit Patent Rights, and Summit Know-How and Sarepta retains all of its rights, title and interests in and to the Sarepta Patent Rights and Sarepta Know-How. Each Party shall own the entire right, title and interest in and to all Know-How (and Patent Rights claiming patentable inventions therein) first made or invented solely by the employees or consultants of such Party in the course of the Collaboration. The Parties shall jointly own or Control all rights, title and interests in and to the Collaboration Technology. Inventorship shall be determined in accordance with U.S. patent Laws.

12.1.1. **Right to Practice Collaboration Technology**. Subject to the rights and licenses granted to, and the obligations of, each Party pursuant to this Agreement, each Party is entitled to exploit and practice Collaboration Technology for all purposes on a worldwide basis and to license Collaboration Technology, in each case, without consent of and without a duty of accounting to the other Party. Each Party will grant and hereby does grant all permissions, consents and waivers with respect to, and all licenses under, the Collaboration Technology, throughout the world, necessary to provide the other Party with such rights of use and exploitation of the Collaboration Technology, and will execute documents as necessary to accomplish the foregoing.

12.1.2. **Disclosure**. Each Party shall promptly disclose to the other Party any invention disclosures, or other similar documents, submitted to it by its employees, agents or independent contractors during the Term describing Collaboration Know-How or any Sarepta Know-How or Summit Know-How made or invented in the course of the Collaboration and shall notify the other Party if it intends to file any patent application disclosing or claiming any such Know-How or invention. In addition, each Party will disclose to the other Party any such information related to such technology, to the extent patentable, necessary for the filing, prosecution or maintenance of any Patent Right Covering Collaboration Know-How, Summit Know-How or Sarepta Know-How in accordance with the terms and conditions of this Article 12.

12.1.3. **Employee Assignment Obligations; Third Party Intellectual Property Agreements**. Each Party shall ensure that all of its employees and all of its Affiliates' employees acting under its or its Affiliates' authority in the performance of this Agreement assign to such Party under a binding written

agreement all Know-How and Patent Rights discovered, made or conceived by such employee as a result of such employee's employment. In addition, each Party shall use Commercially Reasonable Efforts to include in agreements between such Party and its Affiliates, on the one hand, and Third Parties engaged under such agreements to perform activities under this Agreement that are reasonably expected to generate Know-How or Patent Rights, on the other hand, binding agreements granting such Party Control of such generated Know-How and Patent Rights that are reasonably necessary or useful for the Development, Manufacture and Commercialization of Licensed Products hereunder; provided that, in entering into such a Third Party agreement, a Party may, in the exercise of reasonable business judgment, accept less than such rights if such Party determines that such rights cannot be obtained from such Third Party on commercially reasonable terms and that such agreement is nonetheless consistent with and advisable to further the Parties' related Development, Manufacturing and Commercialization goals under this Agreement.

12.2. Prosecution and Maintenance of Patent Rights.

12.2.1. Sarepta Patent Rights. Sarepta has the sole responsibility to, at Sarepta's discretion, file, prosecute and maintain, all Sarepta Patent Rights, in Sarepta's name.

12.2.2. Summit Patent Rights and Collaboration Patent Rights.

(a) Responsibility. Subject to Section 12.2.2(c), Summit has the sole responsibility to, at Summit's discretion, file, prosecute and maintain, all Summit Patent Rights in Summit's name and Collaboration Patent Rights jointly in the name of each Party, in the case of Collaboration Patent Rights, using counsel reasonably acceptable to Sarepta. Summit agrees to use Commercially Reasonable Efforts to prosecute and maintain all Summit Patent Rights and Collaboration Patent Rights throughout the world; [**].

(b) Consultation with Sarepta. Notwithstanding the foregoing Section 12.2.2(a), Summit shall consult with Sarepta on the preparation, filing, prosecution and maintenance of all Summit Patent Rights and Collaboration Patent Rights throughout the world. Summit shall furnish Sarepta with copies of documents relevant to such preparation, filing, prosecution and maintenance in sufficient time prior to filing such document or making any payment due thereunder to allow for review and comment by Sarepta and shall consider in good faith timely comments from Sarepta thereon. Summit shall also furnish Sarepta with copies of all final filings and responses made to any patent authority with respect to all such Patent Rights in a timely manner following submission thereof.

(c) Sarepta Step-In Right. In the event that Summit elects not to file, prosecute or maintain patent protection on any Summit Patent Rights or Collaboration Patent Rights in the Sarepta Territory, subject to the terms and conditions of any applicable Summit In-License, Sarepta shall have the right (but not the obligation), at its expense, to file, prosecute and maintain in any country patent protection on such abandoned Patent Rights in the Sarepta Territory. If Sarepta exercises such step-in right, then (i) Sarepta will control, and have final decision making authority with respect to, the filing, prosecution and maintenance of applicable Summit Patent Rights or Collaboration Patent Rights at its sole cost and expense and (ii) Sarepta shall have the right to offset [**] of all reasonable Out-of-Pocket Costs arising from such prosecution and maintenance against any royalties that become payable to Summit hereunder with respect to Licensed Products Covered by such Summit Patent Rights or Collaboration Patent Rights based on Net Sales in the applicable country(ies) of the Sarepta Territory. In addition, Summit shall use Commercially Reasonable Efforts to make available to Sarepta its authorized attorneys, agents or representatives, and such of its employees, in each case, as are reasonably necessary to assist Sarepta in obtaining and maintaining the patent protection described under this Section 12.2.2. Summit shall sign or use Commercially Reasonable Efforts to have signed all legal documents necessary to file and prosecute such patent applications or to obtain or maintain such patents.

12.2.3. **Cooperation**. Each Party hereby agrees: (a) to make its employees, agents and consultants reasonably available to the other Party (or to the other Party's authorized attorneys, agents or representatives), to the extent reasonably necessary to enable such Party to undertake patent prosecution; (b) to provide the other Party with copies of all material correspondence pertaining to prosecution with the patent offices; (c) to cooperate, if necessary and appropriate, with the other Party in gaining patent term extensions wherever applicable to Patent Rights licensed under this Agreement and (d) to endeavor in good faith to coordinate its efforts with the other Party to minimize or avoid interference with the prosecution and maintenance of the other Party's patent applications.

12.3. **Infringement by Third Parties; Defense Actions**.

12.3.1. **Notices**. Each Party shall promptly report in writing to the other Party any (a) known or suspected infringement of any Summit Technology or Sarepta Technology or (b) unauthorized use or misappropriation of any Confidential Information or Know-How of a Party by a Third Party of which it becomes aware, in each case, to the extent such infringing, unauthorized or misappropriating activities involve, as to a Licensed Product, a competing product in the Field ((a) and (b) collectively, "**Competitive Infringement**"), (c) Third Party's challenge to the validity, scope or enforceability of a Summit

Patent Right, Sarepta Patent Right or Collaboration Patent Right or (d) initiation by a Third Party of any opposition or *inter partes* review proceeding against any Summit Patent Right, Sarepta Patent Right or Collaboration Patent Right (a "**Defense Action**"), and shall provide the other Party with all available evidence and information regarding such Competitive Infringement or Defense Action.

12.3.2. Rights to Enforce and Defend.

(a) Sarepta Territory. Sarepta shall have the sole and exclusive right to initiate an infringement or other appropriate suit or administrative proceeding in the Sarepta Territory against any Third Party as to any Competitive Infringement in the Sarepta Territory of any Sarepta Technology, and, Sarepta shall have the first right, but not the obligation, to initiate an infringement or other appropriate suit or administrative proceeding in the Sarepta Territory against any Third Party as to any Competitive Infringement in the Sarepta Territory of any Summit Technology (subject to the provisions of any Summit In-License) or Collaboration Technology. Likewise, Sarepta will have the first right, but not the obligation, to defend against any Defense Action in the Sarepta Territory relating to a Summit Patent Right (subject to the provisions of any Summit In-License) or Collaboration Patent Right, and will have the sole and exclusive right to defend any Defense Action in the Sarepta Territory relating to a Sarepta Patent Right.

(b) Summit Territory. Summit shall have the sole and exclusive right to initiate an infringement or other appropriate suit or administrative proceeding in the Summit Territory against any Third Party as to any Competitive Infringement in the Summit Territory of any Summit Technology, and Summit shall have the first right, but not the obligation, to initiate an infringement or other appropriate suit or administrative proceeding in the Summit Territory against any Third Party as to any Competitive Infringement in the Summit Territory of any Sarepta Technology (subject to the provisions of any Sarepta In-License) or Collaboration Technology. Likewise, Summit will have the sole and exclusive right, but not the obligation, to defend against any Defense Action in the Summit Territory relating to the Summit Patent Rights and shall have the first right, but not the obligation, to defend any Defense Action in the Summit Territory relating to the Collaboration Patent Rights or Sarepta Patent Rights (subject to the provisions of any Sarepta In-License).

(c) Step-In Right. If within [**] after a Party's receipt of a notice of a Competitive Infringement or Defense Action (or such lesser time so that the other Party's rights are not prejudiced by the delay) with respect to which such Party has the first right (but not sole and exclusive right) to initiate an infringement or other appropriate suit or

administrative proceeding as to such Competitive Infringement or to defend such Defense Action, such Party does not take any action as described in Section 12.3.2(a) or Section 12.3.2(b) and permitted hereunder against such Competitive Infringement or in defense of such Defense Action, then the other Party may in its sole discretion, bring and control any legal action in connection with such Competitive Infringement or Defense Action at its sole expense, subject to the provisions of any applicable In-License.

12.3.3. **Procedures; Expenses and Recoveries**. The Party having the right to initiate or defend any suit, action or administrative proceeding to challenge any Competitive Infringement or to defend a Defense Action under Section 12.3.2 shall have the sole and exclusive right to select counsel for any such suit, action or proceeding and shall pay all expenses of the suit, action or proceeding, including attorneys' fees and court costs and reimbursement of the other Party's reasonable Out-of-Pocket Costs in rendering assistance requested by the initiating or defending Party. If required under applicable Law in order for the initiating or defending Party to initiate, defend or maintain such suit, action or proceeding, or if either Party is unable to initiate, prosecute or defend such suit, action or proceeding solely in its own name or it is otherwise advisable to obtain an effective legal remedy, in each case, the other Party shall join as a party to the suit, action or proceeding and will execute and cause its Affiliates to execute all documents necessary for the initiating or defending Party to initiate, maintain or defend such suit, action or proceeding. In addition, at the initiating or defending Party's request, the other Party shall provide reasonable assistance to the initiating or defending Party in connection with such suit, action or proceeding at no charge to the initiating or defending Party except for reimbursement by the initiating or defending Party of reasonable Out-of-Pocket Costs incurred in rendering such assistance. The non-initiating or non-defending Party shall have the right to participate and be represented in any such suit, action or proceeding by its own counsel at its own expense. If the Parties obtain from a Third Party, in connection with such a suit, action or proceeding, any damages, license fees, royalties or other compensation (including any amount received in settlement of such litigation or the applicable dispute), then such amounts shall be allocated in all cases as follows:

(a) first, to reimburse each Party for all expenses of the suit incurred by the Parties, including attorneys' fees and disbursements, court costs and other litigation expenses;

(b) second, [**] of the balance to be paid to Sarepta with respect to enforcement of the Sarepta Technology in the Sarepta Territory or to Summit with respect to enforcement of the Summit Technology in the Summit Territory; and

(c) third, [**] of the balance to be paid to the Party initiating the suit and [**] of the balance to be paid to the other Party.

12.3.4. **Settlement.** Neither Party will enter into any settlement of any an infringement or other appropriate suit or administrative proceeding against a Competitive Infringement or any Defense Action that could reasonably be expected to materially adversely affect the other Party's rights or interests without such other Party's written consent, which consent will not be unreasonably withheld.

12.4. **Patent Term Extensions**. Subject to the provisions of any Summit In-License, Summit shall use Commercially Reasonable Efforts to obtain all available supplementary protection certificates ("**SPC**") and other extensions of Summit Patent Rights in the Sarepta Territory. If more than one patent is eligible for extension or patent term restoration in the Sarepta Territory, then the Parties will use good faith efforts to mutually agree on a strategy with the goal of maximizing patent protection and commercial value for the Licensed Product, and, subject to the provisions of any Summit In-License, Summit will seek patent term extensions, restorations and SPCs in accordance with that strategy. Sarepta will execute such authorizations and other documents and take such other actions as may be reasonably requested by Summit to obtain any such extensions, restorations and SPCs.

12.5. **Common Interest**. All information exchanged between the Parties' representatives regarding the preparation, filing, prosecution, maintenance, enforcement or defense of the Patents Rights under this Section 12 will be deemed Confidential Information. In addition, the Parties acknowledge and agree that, with regard to such preparation, filing, prosecution, maintenance and enforcement of the Patents Rights under this Section 12, the interests of the Parties as collaborators and licensor and licensee are to obtain the strongest patent protection possible, and as such, are aligned and are legal in nature. The Parties agree and acknowledge that they have not waived, and nothing in this Agreement constitutes a waiver of, any legal privilege concerning the Patents Rights under this Section 12, including privilege under the common interest doctrine and similar or related doctrines.

12.6. **EU Unitary Patent System**. Without limitation of Sarepta's rights under this Section 12, Sarepta shall have the exclusive right to opt-in and opt-out the Sarepta Patent Rights, Summit Patent Rights (subject to the provisions of any Summit In-License) and Collaboration Patent Rights from the jurisdiction of the E.U. Unified Patent Court, in accordance with Unified Patent Court (Regulation (E.U.) No. 1257/2012) and its applicable Annexes and Rules of Procedure, as amended and from time to time in effect, and Summit shall not do so.

12.7. **Third Party Infringement Claims.** If a Third Party sues a Party alleging that the sued Party's, or the sued Party's Sublicensee's, Development, Manufacture or Commercialization of a Licensed Product infringes or will infringe said Third Party's intellectual property, then upon the sued Party's request and in connection with the sued Party's defense of any such Third Party suit, the other Party will provide reasonable

assistance to the sued Party for such defense. The sued Party will keep the other Party, if such other Party has not joined in such suit, reasonably informed regarding such suit on a quarterly basis, in person or by telephone, prior to and during the pendency of any such suit.

12.8. Trademarks.

12.8.1. Use of Trademarks in Each Party's Territory. Each Party has the right to use any trademark it owns or controls (other than by virtue of a license under this Section 12.8) for Licensed Products in its Territory at its sole discretion, and each Party and its Affiliates shall retain all rights, title and interests in and to its and their respective corporate names and logos.

12.8.2. Product Trademarks. Sarepta will develop and propose, and the JSC shall review and comment on, one or more Product Trademark(s) for use by Sarepta and its Related Parties throughout the Sarepta Territory. Such Product Trademark(s) considered by the JSC may include the Product Trademark(s) developed or used by Summit with respect to the Commercialization of Licensed Products in the Summit Territory (the "**Summit Trademarks**"). Any Product Trademark(s) (other than the Summit Trademarks) that are used by Sarepta to Commercialize Licensed Products in the Sarepta Territory are hereinafter referred to as the "**Sarepta Trademarks**." Summit (or its Related Parties, as appropriate) shall own all rights to Summit Trademarks, and all goodwill associated therewith, throughout the Summit Territory and the Sarepta Territory. Sarepta (or its Related Parties, as appropriate) shall own all rights to Sarepta Trademarks and all goodwill associated therewith, throughout the Sarepta Territory and Summit Territory. Summit shall also own rights to any Internet domain names incorporating the applicable Summit Trademarks or any variation or part of such Summit Trademarks used as its URL address or any part of such address; and Sarepta shall also own rights to any Internet domain names incorporating the applicable Sarepta Trademarks or any variation or part of such Sarepta Trademarks used as its URL address or any part of such address.

12.8.3. Sarepta's Use of Summit Trademarks. If Sarepta or its Related Parties use any Summit Trademarks to Commercialize any Licensed Product in the Sarepta Territory, then the following provisions shall apply: Summit shall and hereby does grant to Sarepta an exclusive royalty-free, fully paid-up, irrevocable, perpetual license to use the applicable Summit Trademark(s) and the goodwill associated therewith to Commercialize such Licensed Product in the Sarepta Territory. Sarepta agrees that the quality of such Licensed Product and the Manufacture and Commercialization thereof shall be consistent with the quality standards applied by Summit thereto. In addition, Sarepta shall comply strictly with Summit's trademark style and usage standards that Summit provides to Sarepta in writing from time to time with respect to the Summit Trademarks. Sarepta shall at its own expense, at the request of Summit from time to time, submit to Summit for approval a reasonable number of

production samples of such Licensed Product and related packaging materials. In the event that Summit reasonably objects to the quality of such Licensed Product or the usage of the Summit Trademarks in connection with any sample, it shall give written notice of such objection to Sarepta within sixty (60) days of receipt by Summit of the sample, specifying the way in which such usage of its Summit Trademarks fails to meet the style, usage or quality standards for such Licensed Product set forth in the first two sentences of this Section 12.8.3, and Sarepta shall immediately cease sale and distribution of such Licensed Product. If Sarepta wishes to continue to distribute and sell such Licensed Product, then it must remedy the failure and submit further samples to Summit for approval.

12.8.4. **Summit's Use of Sarepta Trademarks**. Neither Summit nor its Related Parties shall use any Sarepta Trademarks to Commercialize any Licensed Product in the Summit Territory; provided that, Sarepta does not adopt any Sarepta Trademark that is confusingly similar to or incorporates any Summit Trademark.

12.8.5. **Maintenance and Enforcement**. If Summit Trademarks are used to Commercialize any Licensed Product in the Sarepta Territory, then Summit will use Commercially Reasonable Efforts to establish, maintain, enforce and defend such Summit Trademarks in the applicable countries of the Sarepta Territory during the Term. Sarepta shall be responsible for [**] of the costs of such efforts in the Sarepta Territory and Sarepta shall reimburse Summit for all such costs incurred by Summit within forty-five (45) days after receiving any invoice from Summit for such costs. Sarepta will use Commercially Reasonable Efforts to establish, maintain, enforce and defend any Sarepta Trademarks in the Sarepta Territory during the Term, at its expense for so long as they are being used in connection with Licensed Products.

12.8.6. **Product Trademark Infringement**. In the event either Party becomes aware of any infringement of any Product Trademark by a Third Party, such Party shall promptly notify the other Party and the Parties shall consult with each other and jointly determine the best way to prevent such infringement, including by the institution of legal proceedings against such Third Party.

12.8.7. **Use of Names**. For the avoidance of doubt, neither Party shall have any right to use the other Party's or the other Party's Affiliates' corporate names or logos in connection with Commercialization of Licensed Products.

12.9. **Acknowledgment**. It is the intention of the Parties that this Agreement is a "joint research agreement" pursuant to Section 35 U.S.C. 102(c).

13. TERM AND TERMINATION

13.1. **Term**. This Agreement shall be effective as of the Effective Date and, unless terminated earlier pursuant to Section 13.2, this Agreement shall continue in effect on a Licensed

Product-by-Licensed Product and country-by-country basis until expiration of the last Royalty Term to expire under this Agreement ("**Term**"). Upon expiration of the Term, all licenses granted to Sarepta under Section 7.1 then in effect shall become fully paid-up, perpetual, irrevocable licenses and all licenses granted to Summit under Section 7.2 then in effect shall become fully paid-up, perpetual, irrevocable licenses.

13.2. Termination Rights.

13.2.1. Termination for Convenience.

(a) *By Sarepta*. Sarepta shall have the right to terminate this Agreement in its entirety or on a Licensed Product-by-Licensed Product or country-by-country basis at any time after the Effective Date on six (6) months' prior written notice to Summit. If Sarepta terminates this Agreement with respect to one or more, but not all, countries in the Sarepta Territory, then those countries will cease being part of the Sarepta Territory commencing on the effective date of such termination.

(b) *Summit's Resulting Right*. If any termination by Sarepta pursuant to Section 13.2.1(a) prevents Sarepta from satisfying its obligations with respect to the Terminated Licensed Product in the Major European Countries as set forth in Sections 3.3 and 4.1.1, then such termination shall give Summit the right to terminate this Agreement with respect to such Terminated Licensed Product as to the EU by providing written notice to Sarepta within [**] of Summit's receipt of Sarepta's notice of termination pursuant to Section 13.2.1(a). If any termination by Sarepta pursuant to Section 13.2.1(a) prevents Sarepta from satisfying its obligations with respect to the Terminated Licensed Product in the Major Option Countries as set forth in Sections 3.3 and 4.1.1, then such termination shall give Summit the right to terminate this Agreement with respect to such Terminated Licensed Product as to the Option Territory by providing written notice to Sarepta within [**] of Summit's receipt of Sarepta's notice of termination pursuant to Section 13.2.1(a). The effects of such termination will be as if Summit terminated this Agreement with respect to such Terminated Licensed Product in the EU or the Option Territory, as applicable, pursuant to Section 13.2.2.

13.2.2. Termination for Cause. This Agreement may be terminated, in its entirety or on a Licensed Product-by-Licensed Product basis, at any time during the Term upon written notice by either Party if the other Party is in material breach of its obligations hereunder and has not cured such breach within [**] in the case of a payment breach, or within [**] in the case of all other breaches, after notice requesting cure of the breach; provided, however, that if any breach other than a payment breach is not reasonably curable within [**] and if a Party is making a *bona fide* effort to cure such breach, then such termination shall be delayed for a time period to be agreed by both Parties, not to exceed an additional [**],

in order to permit such Party a reasonable period of time to cure such breach. For the avoidance of doubt, any failure by Sarepta to satisfy its diligence obligations in Sections 2.4, 3.3 or 4.1.1 with respect to a Licensed Product shall be deemed a material breach only with respect to such Licensed Product for purposes of this Section 13.2.2. If Sarepta fails to satisfy its diligence obligations in Sections 3.3 or 4.1.1 as to a Licensed Product in the Major European Countries or, if applicable, the Major Option Countries, then Summit shall have the right to terminate this Agreement as to such Licensed Product only in the EU or the Option Territory, as applicable, as a result of such failure. If Sarepta fails to satisfy its diligence obligations in Sections 3.3 or 4.1.1 as to any country in the Sarepta Territory outside the EU and the Option Territory, then Summit shall have the right to terminate this Agreement as to such Licensed Product only in such country as a result of such failure. If either Party initiates a dispute resolution procedure in accordance with Section 14.3.2 to resolve a dispute, claim or controversy regarding the material breach for which termination is being sought and is diligently pursuing such procedure, then the cure period set forth in this Section 13.2.2 will be tolled during the pendency of such dispute resolution procedure.

13.2.3. Termination for Safety Reasons.

(a) Termination by Sarepta. At any time during the [**] after the Effective Date, Sarepta may terminate this Agreement with respect to the Benzoxazole Licensed Product on not less than [**] prior written notice to Summit if Sarepta reasonably determines based upon its review of the clinical data or upon a determination by an applicable drug safety monitoring board or Governmental Authority that the Benzoxazole Licensed Product caused or is likely to cause a fatal, life-threatening or other Serious Adverse Event that is reasonably expected, based upon then-available data, to preclude continued Development or Commercialization of the Benzoxazole Licensed Product (such termination, a "**Safety Termination**"). Upon delivery of any such notice of a Safety Termination, each Party may wind-down its then on-going activities related to the Benzoxazole Licensed Product, including any on-going Clinical Studies, in accordance with Section 13.3.2(c)(ii) (to the extent consistent with applicable Laws).

(b) Termination by Consensus. The Parties may terminate this Agreement on a Licensed Product-by-Licensed Product basis prior to expiration of the [**] notice period provided in Section 13.3.2(a) upon written agreement if the Parties: (i) reach consensus that the Party proposing the Safety Termination is unable to continue Developing or Commercializing a Licensed Product in the Field in its Territory; and (ii) have completed all applicable wind-down and other transition activities, including those set forth in Section 13.3.2(c)(ii).

13.2.4. **Challenges of Patent Rights**. In the event that Sarepta or any of its Related Parties (a) commences or participates in any action or proceeding (including any patent opposition or re-examination proceeding), or otherwise asserts any claim, challenging or denying the validity or enforceability of any Summit Patent Right or any claim thereof or (b) actively assists any other Person in bringing or prosecuting any action or proceeding (including any patent opposition or re-examination proceeding) challenging or denying the validity or enforceability of any of such Summit Patent Rights or any claim thereof, then (i) Sarepta shall give notice thereof to Summit within [**] of taking such action (or becoming aware that its Related Party has taken such action) and (ii) Summit will have the right, in its sole discretion to give notice to Sarepta that the licenses granted to Sarepta with respect to all or any portion of the Summit Patent Rights licensed to Sarepta under this Agreement will terminate [**] following such notice (or such longer period as Summit may designate in such notice) unless (x) Sarepta withdraws or causes to be withdrawn all such challenge or (y) in the case of *ex-parte* proceedings, multi-party proceedings or other patent challenges that Sarepta or Sarepta's Related Parties do not have the power to unilaterally withdraw or cause to be withdrawn, Sarepta and Sarepta's Related Parties cease assisting any other party to such patent challenge and, to the extent Sarepta or a Sarepta Related Party is a party to such patent challenge, it withdraws from such patent challenge, in each case, within such [**]day period. In the event that Summit is not permitted under Law to terminate the licenses with respect to all Summit Patent Rights under this Agreement, then the Parties agree to construe this provision to permit Summit to terminate only the licenses to that portion of such Summit Patent Rights with respect to which Summit may terminate consistent with applicable Law. The foregoing shall not apply with respect to (A) any patent challenge described in clause (a) or (b) above that is made in defense of Summit's assertion of any Summit Patent Right against Sarepta or any of its Related Parties and (B) any patent challenge commenced by a Third Party that after the Effective Date acquires or is acquired by Sarepta or its Related Parties or its or their business or assets, whether by stock purchase, merger, asset purchase or otherwise, provided that such patent challenge commenced prior to the closing of such acquisition.

13.3. **Effect of Termination**.

13.3.1. **Termination by Sarepta for Summit Breach**. Without limiting any other legal or equitable remedies that either Party may have, if Sarepta has the right to terminate this Agreement pursuant to Section 13.2.2 in its entirety or with respect to a particular Licensed Product(s) (in the form such Licensed Product exists as of the effective date of termination, a "**Terminated Licensed Product**"), then Sarepta may elect, upon written notice to Summit, to either:

(a) Termination. Terminate this Agreement in its entirety, in which case:

(i) all license grants in this Agreement from either Party to the other shall immediately terminate;

(ii) Sarepta shall as promptly as practicable transfer to Summit or Summit's designee (x) possession and ownership of all governmental or regulatory correspondence, conversation logs, filings and approvals (including all Regulatory Approvals and pricing and reimbursement approvals) relating to the Development, Manufacture or Commercialization of the Terminated Licensed Product and all Sarepta Trademarks used for the applicable Terminated Licensed Product(s) in the Field in the Sarepta Territory (but not any Sarepta house marks or any trademark containing the word "Sarepta" owned by Sarepta and used for the Terminated Licensed Products in the Field in the Sarepta Territory), (y) copies of all data, reports, records and materials, and other sales and marketing related information in Sarepta's possession or Control to the extent that such data, reports, records, materials or other information relate to the Development, Manufacture or Commercialization of the Terminated Licensed Product, including all non-clinical and clinical data relating to the Terminated Licensed Product, and customer lists and customer contact information and all adverse event data in Sarepta's possession or Control; provided that Sarepta shall use Commercially Reasonable Efforts to obtain for Summit the right to access all such data, reports, records, materials and other sales and marketing related information and (z) all records and materials in Sarepta's possession or Control containing Confidential Information of Summit. Sarepta shall further appoint Summit as Sarepta's or Sarepta's Related Parties' agent for all Licensed Product-related matters involving Regulatory Authorities in the Sarepta Territory until all Regulatory Approvals and other regulatory filings have been transferred to Summit or its designee; and

(iii) Sarepta shall cease to have any financial obligations under this Agreement (including obligations with respect to Development Costs pursuant to Section 2.2.3 or payments pursuant to Article 8); or

(b) Payment Reduction. Maintain this Agreement in full force and effect (foregoing the right to terminate this Agreement for such occurrence of such breach) and all amounts set forth in Article 8 that are thereafter owed by Sarepta to Summit shall be reduced by [**]. In addition to the reduction set forth in this Section 13.3.1(b), the consequences set forth in Section 13.4 shall also apply in the circumstances set forth therein.

13.3.2. **Termination for Safety Reasons; Termination by Summit for Sarepta Breach or Patent Challenge or by Sarepta for Convenience**. Without limiting any other legal or equitable remedies that either Party may have, if this Agreement is terminated, in its entirety or with respect to a Terminated Licensed Product, by Sarepta under Section 13.2.1 or Section 13.2.3, or by Summit under Section 13.2.2, Section 13.2.3 or Section 13.2.4, then:

(a) Milestone Payment. Irrespective of the termination of this Agreement, Sarepta shall make the non-refundable, non-creditable milestone payment to Summit set forth in TABLE 8.2.1(i) no later than the later of (i) April 1, 2017 or (ii) forty-five (45) days after the earliest date on which such milestone event has first been achieved with respect to the first Licensed Product to achieve such milestone event.

(b) License Grant to Summit. The license grants to Summit with respect to the applicable Terminated Licensed Product(s) in Section 7.2 shall survive and shall be expanded to include the Sarepta Territory (or if the Agreement was only terminated with respect to some countries in the Sarepta Territory, those terminated counties (the "**Terminated Countries**")).

(c) On-Going Clinical Trials.

(i) *Completion*. Upon termination of this Agreement in its entirety or with respect to a Licensed Product for any reason listed in this Section 13.3.2 other than pursuant to Section 13.2.3, the Parties may complete any ongoing Clinical Studies relating to the applicable Terminated Licensed Product(s) in the Sarepta Territory. A Clinical Study will be considered "ongoing" if the first patient visit in such Clinical Study had occurred but the last patient visit in such Clinical Study and database lock had not yet occurred at the time notice of termination was delivered.

(ii) *Wind-Down*. Upon Summit's receipt of notice of such termination of the Agreement or the Parties' agreement to terminate pursuant to Section 13.2.3, each Party shall responsibly wind-down, in accordance with accepted pharmaceutical industry norms and ethical practices, any on-going Clinical Studies of the applicable Terminated Licensed Product(s).

(iii) *Responsibilities for Costs*. Upon such termination, Sarepta shall reimburse Summit for forty-five percent (45%) of Summit's Out-of-Pocket Costs incurred following the effective date of such termination in connection with the completion of such ongoing Clinical Studies pursuant to Section 13.3.2(c)(i) or the wind-down of such ongoing Clinical Studies pursuant to

Section 13.3.2(c)(ii). Summit shall invoice Sarepta following the end of each Calendar Quarter for such amounts due under this Section 13.3.2(c)(iii), and shall provide supporting documentation as reasonably requested by Sarepta, and Sarepta shall reimburse Summit for all such costs incurred by Summit within forty-five (45) days after receiving any invoice from Summit for such costs. Sarepta shall have the right to audit Summit's records relating to such Out-of-Pocket Costs in accordance with Section 8.5.

(d) Transfer of Regulatory Materials. At Summit's option, Sarepta shall as promptly as practicable transfer to Summit or Summit's designee (i) possession and ownership of all governmental or regulatory correspondence, conversation logs, filings and approvals (including all Regulatory Approvals and pricing and reimbursement approvals) relating to the Development, Manufacture or Commercialization of the applicable Terminated Licensed Product(s) in the applicable Terminated Country(ies), (ii) copies of all data, reports, records and materials and other sales and marketing related information in Sarepta's possession or Control to the extent that such data, reports, records, materials or other information relate to the Development, Manufacture or Commercialization of the applicable Terminated Licensed Product(s) in the applicable Terminated Country(ies), including all non-clinical and clinical data relating to the applicable Terminated Licensed Product(s), and customer lists and customer contact information and all adverse event data in Sarepta's possession or Control relating to the applicable Terminated Licensed Product(s); provided that Sarepta shall use Commercially Reasonable Efforts to obtain for Summit the right to access all such data, reports, records, materials and other sales and marketing related information and (iii) all records and materials in Sarepta's possession or Control containing Confidential Information of Summit relating to the applicable Terminated Licensed Product(s) in the applicable Terminated Country(ies). Sarepta shall further appoint Summit as Sarepta's or Sarepta's Related Parties' agent for all applicable Terminated Licensed Product(s)-related matters involving Regulatory Authorities in the Sarepta Territory (or the in the applicable Terminated Country(ies), if applicable) until all applicable Regulatory Approvals and other regulatory filings have been transferred to Summit or its designee.

(e) Appointment as Distributor. Upon termination of this Agreement for any reason listed in this Section 13.3.2 other than pursuant to Section 13.2.3, at Summit's option, if the effective date of termination is after First Commercial Sale, then Sarepta shall appoint Summit as its exclusive distributor of the applicable Terminated Licensed Product(s) in the Sarepta Territory (or the in the applicable Terminated Country(ies), if applicable) and grant Summit the right to appoint sub-distributors,

until such time as all applicable Regulatory Approvals in the Sarepta Territory (or the in the applicable Terminated Country(ies), if applicable) have been transferred to Summit or its designee.

(f) Third Party Agreements. Upon termination of this Agreement in its entirety or with respect to a Licensed Product for any reason listed in this Section 13.3.2 other than pursuant to Section 13.2.3, at Summit's option, and to the extent permitted under Sarepta's obligations to Third Parties at the time of termination, Sarepta shall transfer to Summit any Third Party agreements relating solely and exclusively to the Development, Manufacture or Commercialization of the applicable Terminated Licensed Product(s) to which Sarepta is a party, subject to any required consents of such Third Party, which Sarepta shall use Commercially Reasonable Efforts to obtain promptly.

(g) Trademark Assignment. At Summit's option, Sarepta shall promptly transfer and assign to Summit all of Sarepta's and its Affiliates' rights, title and interests in and to the Sarepta Trademark(s) used for the applicable Terminated Licensed Product(s) in the Field in the Sarepta Territory (but not any Sarepta house marks or any trademark containing the word "Sarepta" owned by Sarepta and used for the Terminated Licensed Products in the Field in the Sarepta Territory);

(h) Supply of Terminated Licensed Product. At Summit's option, Sarepta shall transfer to Summit any inventory of the applicable Terminated Licensed Product(s) Controlled by Sarepta or its Affiliates as of the termination date at the actual price paid by Sarepta for such supply.

(i) Further Assistance. Sarepta shall provide any other assistance reasonably requested by Summit for the purpose of allowing Summit or its designee to proceed expeditiously with the Development, Manufacture and Commercialization of the applicable Terminated Licensed Product(s) in the Sarepta Territory. Sarepta shall execute all documents and take all such further actions as may be reasonably requested by Summit in order to give effect to the foregoing clauses.

13.4. Breach of Summit's Development Obligations for Next Generation Product. Without limiting Sarepta's rights under Section 13.2.2 with respect to other material breaches, if Summit abandons substantially all of the activities allocated to it under the Development Plan with respect to the Next Generation Products, and Sarepta does not terminate this Agreement in its entirety for cause pursuant to Section 13.2.2, then, in addition to Sarepta's remedies under Section 13.3.1(b), then Sarepta may elect to assume Summit's Development responsibilities under the Collaboration with respect to such Next Generation Products, and if Sarepta does so assume such responsibilities, Sarepta will be entitled to the following remedies:

13.4.1. Termination of Development Milestones. Sarepta's obligation to pay then-unpaid development milestone fees with respect to Next Generation Products under Section 8.2 shall terminate.

13.4.2. **Tie Breaking Authority**. Notwithstanding Section 5.4.3, the Chief Executive Officer of Sarepta or his or her designee shall have the deciding vote on any matter involving the Development of the Next Generation Products.

13.5. **Effect of Expiration or Termination; Survival**. Expiration or termination of this Agreement shall not relieve the Parties of any obligation accruing prior to such expiration or termination; provided that, subject to Section 13.3.2(a), Sarepta will have no obligation to pay any milestone payments that accrue under Section 8.2 as a result of any milestone achieved thereunder following the date on which any notice of termination of this Agreement is provided. Any expiration or termination of this Agreement shall be without prejudice to the rights of either Party against the other accrued or accruing under this Agreement prior to expiration or termination, including the obligation to pay royalties for the Terminated Licensed Product sold prior to such expiration or termination. The provisions of Sections 1, 7.6, 8.5, 8.6, 8.7, 8.8, 8.9, 9.1, 9.2.1, 10.4, 11, 12.1, 13.3, 13.4.2, 13.5 and 14 shall survive any expiration or termination of this Agreement in accordance with their terms. Except as otherwise set forth in this Section 13, upon termination or expiration of this Agreement all rights and obligations of the Parties under this Agreement shall cease.

14. MISCELLANEOUS

14.1. **Standstill.** For the period beginning on the Effective Date and ending on the date that Regulatory Approval is first received for a Licensed Product (the "**Standstill Period**"), unless the other Party has specifically invited it to do so in writing, neither Party nor any of its Affiliates or representatives acting on behalf of and at the direction of such Party or any of its Affiliates (collectively, the "**Standstill Parties**") will in any manner, directly or indirectly: (a) effect or seek, offer or propose (whether publicly or otherwise) to effect, or cause or participate in or assist or request any other Person to effect or seek, offer or propose (whether public or otherwise) to effect or participate in (i) any acquisition of any securities (or beneficial ownership thereof) or assets of the other Party; (ii) any tender or exchange offer, merger or other business combination involving the other Party; (iii) any recapitalization, restructuring, liquidation, dissolution or other extraordinary transaction with respect to the other Party; or (iv) any "solicitation" of "proxies" (as such terms are used in the proxy rules of the SEC) or consents to vote any voting securities of the other Party; (b) form, join or in any way participate in a "group" (as defined under the Exchange Act) with respect to any securities of the other Party; (c) Act in Concert with any person in relation to voting securities of the other Party; (d) otherwise act, alone or in concert with others, to seek to control or influence the management, Board of Directors or policies of Summit, in each case, for the purpose of effecting a Change of Control; (e) negotiate with or provide any information to any Third Party with respect to, or make any statement or proposal to any Third Party with respect to, or make any public announcement or proposal or offer whatsoever with respect to, or act as a financing source for or otherwise invest in any other Third Parties in connection with, or otherwise

solicit, seek or offer to effect any transactions or actions described, or take any action which would reasonably be expected to obligate the other Party to make a public announcement regarding any of the types of matters set forth in clause (a) above; or (f) enter into any discussions or arrangements with any Third Party with respect to any of the foregoing; provided, however, [**].

14.2. **Assignment**. Except as provided in this Section 14.2, this Agreement may not be assigned or otherwise transferred, nor may any right or obligation hereunder be assigned or transferred, by either Party without the written consent of the other Party. However, either Party may, without the other Party's written consent, assign this Agreement and its rights and obligations hereunder in whole or in part to an Affiliate or to a party that acquires, by or otherwise in connection with, merger, sale of assets or otherwise, all or substantially all of the business of the assigning Party to which the subject matter of this Agreement relates. The assigning Party shall remain responsible for the performance by its assignee of this Agreement or any obligations hereunder so assigned. Any purported assignment in violation of this Section 14.2 shall be null, void and of no legal effect.

14.3. **Governing Law; Arbitration**.

14.3.1. **Governing Law**. This Agreement shall be construed and the respective rights of the Parties determined in accordance with the substantive Laws of the State of New York, excluding any conflicts or choice of law rule or principle that might otherwise refer construction or interpretation of this Agreement to the law of another jurisdiction, and the patent Laws of the relevant jurisdiction without reference to any rules of conflict of laws.

14.3.2. **Arbitration**. Any dispute arising out of or relating to this Agreement that has not been resolved pursuant to Section 5.4 shall be resolved through binding arbitration as follows:

(a) A Party may submit such dispute to arbitration by notifying the other Party, in writing, of such dispute. Within thirty (30) days after receipt of such notice, the Parties shall designate in writing a single arbitrator to resolve the dispute; provided, however, that if the Parties cannot agree on an arbitrator within such thirty (30)-day period, then the arbitrator shall be selected by the Boston, Massachusetts office of the American Arbitration Association (the "**AAA**"). The arbitrator shall not be an Affiliate, employee, consultant, officer, director or stockholder of any Party.

(b) Within thirty (30) days after the designation of the arbitrator, the arbitrator and the Parties shall meet, at which time the Parties shall be required to set forth in writing all disputed issues and a proposed ruling on the merits of each such issue.

(c) The arbitrator shall set a date for a hearing, which shall be no later than forty-five (45) days after the submission of written proposals pursuant

to Section 14.3.2(b), to discuss each of the issues identified by the Parties. The Parties shall have the right to be represented by counsel. Except as provided herein, the arbitration shall be governed by the Commercial Arbitration Rules of the AAA; provided, however, that the Federal Rules of Evidence shall apply with regard to the admissibility of evidence and the arbitration shall be conducted by a single arbitrator.

(d) The arbitrator shall use his or her best efforts to rule on each disputed issue within thirty (30) days after the completion of the hearings described in Section 14.3.2(c). The determination of the arbitrator as to the resolution of any dispute shall be binding and conclusive upon all Parties. All rulings of the arbitrator shall be in writing and shall be delivered to the Parties.

(e) The attorneys' fees of the Parties in any arbitration, fees of the arbitrator, and costs and expenses of the arbitration shall be borne by the Parties as determined by the arbitrator.

(f) Any arbitration pursuant to this Section 14.3.2 shall be conducted in Boston, Massachusetts, U.S. Any arbitration award may be entered in and enforced by any court of competent jurisdiction.

(g) Nothing in this Section 14.3.2 shall be construed as limiting in any way the right of a Party to seek an injunction or other equitable relief with respect to any actual or threatened breach of this Agreement or to bring an action in aid of arbitration. Should any Party seek an injunction or other equitable relief, or bring an action in aid of arbitration, then for purposes of determining whether to grant such injunction or other equitable relief, or whether to issue any order in aid of arbitration, the dispute underlying the request for such injunction or other equitable relief, or action in aid of arbitration, may be heard by the court in which such action or proceeding is brought.

14.4. **Entire Agreement; Amendments**. This Agreement contains the entire understanding of the Parties with respect to the subject matter hereof, and supersedes all previous arrangements with respect to the subject matter hereof, whether written or oral. This Agreement (including the Exhibits and Schedules hereto) may be amended, or any term hereof modified, only by a written instrument duly-executed by authorized representatives of both Parties.

14.5. **Severability**. If any provision hereof should be held invalid, illegal or unenforceable in any respect in any jurisdiction, then the Parties shall substitute, by mutual consent, valid provisions for such invalid, illegal or unenforceable provisions, which valid provisions in their economic effect are sufficiently similar to the invalid, illegal or unenforceable provisions that it can be reasonably assumed that the Parties would have entered into this Agreement with such valid provisions. In case such valid provisions cannot be agreed upon, the invalid, illegal or unenforceable of one or several provisions of this Agreement

shall not affect the validity of this Agreement as a whole, unless the invalid, illegal or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without such invalid, illegal or unenforceable provisions.

14.6. **Headings**. The captions to the Sections hereof are not a part of this Agreement, but are merely for convenience to assist in locating and reading the several Sections hereof.

14.7. **Waiver of Rule of Construction**. Each Party has had the opportunity to consult with counsel in connection with the review, drafting and negotiation of this Agreement. Accordingly, the rule of construction that any ambiguity in this Agreement shall be construed against the drafting Party shall not apply.

14.8. **Interpretation**. Except where the context expressly requires otherwise, (a) the use of any gender herein shall be deemed to encompass references to either or both genders, and the use of the singular shall be deemed to include the plural (and vice versa), (b) the words "include", "includes" and "including" shall be deemed to be followed by the phrase "without limitation", (c) the word "will" shall be construed to have the same meaning and effect as the word "shall," (d) any definition of or reference to any agreement, instrument or other document herein shall be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein), (e) any reference herein to any Person shall be construed to include the Person's successors and permitted assigns, (f) the words "herein", "hereof" and "hereunder," and words of similar import, shall be construed to refer to this Agreement in its entirety and not to any particular provision hereof, (g) all references herein to Sections, Exhibits or Schedules shall be construed to refer to Sections, Exhibits or Schedules of this Agreement, and references to this Agreement include all Exhibits and Schedules hereto, (h) the word "notice" means notice in writing (whether or not specifically stated) and shall include notices, consents, approvals and other written communications contemplated under this Agreement, (i) provisions that require that a Party, the Parties or any committee hereunder "agree," "consent" or "approve" or the like shall require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise (but excluding e-mail and instant messaging), (j) references to any specific law, rule or regulation, or article, section or other division thereof, shall be deemed to include the then-current amendments thereto or any replacement or successor law, rule or regulation thereof and (k) the term "or" shall be interpreted in the inclusive sense commonly associated with the term "or."

14.9. **No Implied Waivers; Rights Cumulative**. No failure on the part of Summit or Sarepta to exercise, and no delay in exercising, any right, power, remedy or privilege under this Agreement, or provided by statute or at Law or in equity or otherwise, shall impair, prejudice or constitute a waiver of any such right, power, remedy or privilege or be construed as a waiver of any breach of this Agreement or as an acquiescence therein, nor shall any single or partial exercise of any such right, power, remedy or privilege preclude any other or further exercise thereof or the exercise of any other right, power, remedy or privilege.

14.10. **Notices**. All notices which are required or permitted hereunder shall be in writing and sufficient if delivered personally, sent by email, sent by facsimile (and promptly confirmed by personal delivery, registered or certified mail or overnight courier), sent by nationally-recognized overnight courier or sent by registered or certified mail, postage prepaid, return receipt requested, addressed as follows:

If to Summit, to:	85b Park Drive Milton Park, Abingdon Oxfordshire, OX14 4RY United Kingdom Attention: Chief Executive Officer Facsimile No.: +44 1235 443 999
With a copy to:	WilmerHale LLP 60 State Street Boston, MA 02109 Attention: Steven D. Barrett, Esq. Facsimile No.: (617) 526-5000
If to Sarepta, to:	Sarepta Therapeutics 215 First Street, Suite 415 Cambridge, MA 02142 Attention: General Counsel: Ty Howton Email: thowton@sarepta.com
With a copy to:	Ropes & Gray LLP Prudential Tower 800 Boylston Street Boston, MA 02199-3600 Attention: David M. McIntosh Facsimile No.: (617) 235-0507

or to such other address as the Party to whom notice is to be given may have furnished to the other Party in writing in accordance herewith. Any such notice shall be deemed to have been given: (a) when delivered if personally delivered or sent by facsimile on a business day (or if delivered or sent on a non-business day, then on the next business day); (b) on receipt if sent by overnight courier or (c) on receipt if sent by mail.

14.11. **Compliance with Export Regulations**. Neither Party shall export any technology licensed to it by the other Party under this Agreement except in compliance with U.S. export Laws and regulations.

14.12. **Force Majeure**. Neither Party shall be held liable to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in performing any obligation under this Agreement to the extent that such failure or delay is caused by or

results from causes beyond the reasonable control of the affected Party, potentially including embargoes, war, acts of war (whether war be declared or not), insurrections, riots, civil commotions, strikes, lockouts or other labor disturbances, fire, floods or other acts of God. The affected Party shall notify the other Party of such force majeure circumstances as soon as reasonably practical, and shall promptly undertake all reasonable efforts necessary to cure such force majeure circumstances.

14.13. **Independent Contractors**. It is expressly agreed that Summit and Sarepta shall be independent contractors and that the relationship between Summit and Sarepta shall not constitute a partnership, joint venture or agency. Summit shall not have the authority to make any statements, representations or commitments of any kind, or to take any action, that would be binding on Sarepta, without the prior written consent of Sarepta, and Sarepta shall not have the authority to make any statements, representations or commitments of any kind, or to take any action, that would be binding on Summit without the prior written consent of Summit.

14.14. **Counterparts**. The Agreement may be executed in two or more counterparts, including by facsimile or PDF signature pages, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

14.15. **Binding Effect; No Third Party Beneficiaries**. As of the Effective Date, this Agreement shall be binding upon and inure to the benefit of the Parties and their respective permitted successors and permitted assigns. Except as expressly set forth in this Agreement, no Person other than the Parties and their respective Affiliates and permitted assignees hereunder shall be deemed an intended beneficiary hereunder or have any right to enforce any obligation of this Agreement.

[THE REMAINDER OF THIS PAGE HAS BEEN LEFT INTENTIONALLY BLANK]

IN WITNESS WHEREOF, the Parties have executed this Agreement as of the Effective Date.

SAREPTA THERAPEUTICS, INC.

BY: /s/ Edward M. Kaye
NAME: Edward M. Kaye, M.D.
TITLE: President and Chief Executive Officer

SUMMIT (OXFORD) LTD

BY: /s/ Glyn O. Edwards
NAME: Glyn O. Edwards
TITLE: Chief Executive Officer

[Signature Page to License and Collaboration Agreement]

EXHIBIT A

INITIAL DEVELOPMENT PLAN

SCHEDULE 1.34

EXISTING SUMMIT IN-LICENSES

University of Oxford Option Agreement

Schedule 1.64

OPTION DATA PACKAGE

Clinical

1. [**]
2. [**]
3. [**]
4. [**]
5. [**]

Preclinical

To the extent not previously provided to Sarepta:

1. [**]
2. [**]
3. [**]

Manufacturing

To the extent not previously provided to Sarepta:

1. [**]
2. [**]

Other

1. [**]
2. [**]
3. [**]

SCHEDULE 1.89

SUMMIT PATENT RIGHTS

[**]

SCHEDULE 6.2

SUPPLY AGREEMENT TERMS

1. Overview. The Supply Agreements for a Collaboration Compound or Licensed Product may, subject to the planned Development and Commercialization activities of the Parties hereunder, provide for the Manufacture and supply of such product in API Bulk Drug Substance, Bulk Drug Product or Finished Drug Product form (each a "**Product**" and collectively "**Products**") on commercially reasonable terms customary to Third Party contract manufacturing organization supply agreements for pharmaceuticals that are consistent with the principles set forth below. Subject to the foregoing, Summit shall Manufacture and supply, either itself or on a subcontracted basis through a Third Party manufacturer, and Sarepta shall purchase from Summit its requirements of clinical supply of placebo and each Product in accordance with the terms of the Clinical Supply Agreement (which shall be consistent with the principles set forth in this Schedule 6.2). Subject to the foregoing, Summit shall Manufacture and supply, either itself or on a subcontracted basis through a Third Party manufacturer, commercial supply of each Product in accordance with the terms of the Commercial Supply Agreement (which shall be consistent with the principles set forth in this Schedule 6.2).

    a. "**API Bulk Drug Substance**" means a Collaboration Compound in bulk form manufactured for use as an active pharmaceutical ingredient.

    b. "**Bulk Drug Product**" means formulated API Bulk Drug Substance, in bulk form prior to filling and finishing.

    c. "**Finished Drug Product**" means the finished product formulation of a Licensed Product, containing API Bulk Drug Substance, filled into unit packages for final labeling and packaging, and as finally labeled and packaged in a form ready for administration.

2. Supply of Product.

    a. Supply Obligation. Summit will supply placebo and API Bulk Drug Substance, Bulk Drug Product or Finished Drug Product, as applicable, in accordance with the Supply Agreements to be negotiated by the Parties in accordance with Section 6.2 of the Agreement and consistent with the principles set forth in this Schedule 6.2. Sarepta will purchase such placebo and Product (in the product form elected by Sarepta) exclusively from Summit or Summit's subcontracted Third Party manufacturer (and will not obtain or otherwise purchase from any others) (i) unless a Third Party manufacturer has been established as a Second Source or Back-Up Source for Manufacture of Product, in which case Sarepta will purchase Licensed Product (in the Product form elected by Sarepta) from Summit or such Third Party manufacturer or (ii) except as set forth in the applicable Supply Agreement.

b. Supply Price. Sarepta shall pay to Summit a per unit price with respect to supply of placebo and Product "**Supply Price**," which Supply Price will be equivalent to the following:

i. Product that Summit Does Not Manufacture Itself. Any placebo or Product manufactured by Summit's Third Party manufacturers and supplied by Summit to Sarepta will be supplied at a price equal to [**].

ii. Product that Summit Manufactures Itself. Any placebo or Product Manufactured by Summit and supplied to Sarepta will be supplied at a price equal to [**].

e. Specifications. The Supply Agreements will contain agreed written specifications for the API Bulk Drug Substance, Bulk Drug Product and Finished Drug Product, as applicable, and the Clinical Supply Agreement will contain specification for placebo.

4. Term and Termination. The term and termination provisions of each Supply Agreement shall give due consideration to the term and termination provisions of Summit's agreements with its applicable Third Party manufacturers.

5. Compliance, Warranties, Acceptance, Recalls, Indemnification and Limitations of Liability. The Supply Agreements will each contain terms and conditions regarding compliance with Laws (including cGMPs and the Regulatory Approvals for the applicable Product) and specifications for the applicable Product, delivery, acceptance, recalls, indemnification and limitations of liability that are customary in Third Party contract manufacturing agreements. Notwithstanding any provision of this Schedule 6.2, Summit shall not have obligations under the Clinical Supply Agreement with respect to a Collaboration Compound or Licensed Product that are greater than the applicable Third Party manufacturer's obligations to Summit under the applicable agreement with such Third Party manufacturer for such Collaboration Compound or Licensed Product.

6. Shortages, Inventory. The Supply Agreements will each provide that, in the event Summit or its subcontracted Third Party manufacturer is unable to supply placebo or a Product to meet the demand in the combined Summit Territory and Sarepta Territory, then it will allocate placebo or such Product between the two Territories equitably based on anticipated demand.

7. Miscellaneous. The Supply Agreements will each contain other customary terms and provisions for agreements of their type as mutually agreed by the Parties.

SCHEDULE 9.2.3

JOINT PRESS RELEASE

Sarepta Therapeutics and Summit Enter Into Exclusive License and Collaboration Agreement for European Rights to Summit's Utrophin Modulator Pipeline for the Treatment of Duchenne Muscular Dystrophy

- **Sarepta and Summit collaborate to advance the development of novel therapies for patients with Duchenne muscular dystrophy**
- **Summit receives $40 million upfront, with potential future ezutromid-related milestone payments totalling up to $522 million plus royalties**
- **Sarepta and Summit to share research and development costs**
- **Sarepta also receives option for Latin American rights**

Cambridge, MA, and Oxford, UK, 4 October 2016 – Sarepta Therapeutics (NASDAQ: SRPT) and Summit Therapeutics plc (NASDAQ: SMMT, AIM: SUMM) today announced that they have entered into an exclusive license and collaboration agreement granting Sarepta rights in Europe, as well as in Turkey and the Commonwealth of Independent States ('the licensed territory'), to Summit's utrophin modulator pipeline, including its lead clinical candidate, ezutromid, for the treatment of Duchenne muscular dystrophy ('DMD'). As part of the agreement, Sarepta also obtains an option to license Latin American rights to Summit's utrophin modulator pipeline. Summit retains commercialization rights in all other countries.

Utrophin modulation is a potential disease-modifying treatment for all patients with the fatal muscle wasting disease DMD, regardless of their underlying dystrophin gene mutation. Ezutromid is currently in a Phase 2 proof of concept trial called PhaseOut DMD.

"This partnership with Summit Therapeutics furthers our commitment to invest in innovative approaches to treating Duchenne and supports our common goal of improving the lives of patients with DMD," said Edward Kaye, M.D., Sarepta's Chief Executive Officer. "Summit's utrophin modulation technology represents a potentially promising approach to treat DMD, which may complement our current approach of exon skipping therapy."

"Sarepta Therapeutics has paved the way in the development of disease-modifying therapies for DMD with the first FDA-approved drug in this disease area, making them a strong strategic partner to support our utrophin modulator pipeline," commented Glyn Edwards, Chief Executive Officer of Summit. "This agreement provides us with access to Sarepta's development, regulatory and commercialisation expertise for the continued advancement of our promising utrophin modulator pipeline. We look forward to this partnership and working together to bring great advances to patients and families living with DMD."

Under the terms of the agreement, Summit will receive an upfront fee of $40 million. In addition, Summit will be eligible for future ezutromid related development, regulatory and sales milestone payments totalling up to $522 million, including a $22 million milestone upon the first dosing of the last patient in Summit's PhaseOut DMD trial, and escalating royalties ranging from a low to high teens percentage of net sales in the licensed territory. Summit will also be eligible to receive development and regulatory milestones related to its next-generation utrophin modulators. Sarepta and Summit will share specified utrophin modulator-related research and development costs at a 45%/55% split, respectively, beginning in 2018. If Sarepta elects to exercise its option for Latin American rights, Summit would be entitled to additional fees, milestones and royalties.

Sarepta and Summit will host an update call for the Duchenne community on Monday, October 10 at 12:00 EDT. Details of the call can be accessed by visiting http://www.parentprojectmd.org/communitycall.

This announcement contains inside information for the purposes of Article 7 of EU Regulation 596/2014 (MAR).

About Utrophin Modulation in DMD

DMD is a progressive muscle wasting disease that is caused by different genetic faults in the gene that encodes dystrophin, a protein that is essential for the healthy function of all muscles. There is currently no cure for DMD and life expectancy is into the late twenties. Utrophin protein is functionally and structurally similar to dystrophin. In preclinical studies, the continued expression of utrophin has a meaningful, positive effect on muscle performance. Summit believes that utrophin modulation has the potential to treat all patients with DMD, regardless of the underlying dystrophin gene mutation. Summit also believes that utrophin modulation could potentially be complementary to other therapeutic approaches for DMD. The Company's lead utrophin modulator, ezutromid, is an orally administered, small molecule. DMD is an orphan disease, and the US Food and Drug Administration ('FDA') and the European Medicines Agency have granted orphan drug status to ezutromid. Orphan drugs receive a number of benefits including additional regulatory support and a period of market exclusivity following approval. In addition, ezutromid has been granted Fast Track designation and Rare Pediatric Disease designation by the FDA.

About Summit Therapeutics

Summit is a biopharmaceutical company focused on the discovery, development and commercialisation of novel medicines for indications for which there are no existing or only inadequate therapies. Summit is conducting clinical programmes focused on the genetic disease Duchenne muscular dystrophy and the infectious disease *C. difficile* infection. Further information is available at www.summitplc.com and Summit can be followed on Twitter (@summitplc).

About Sarepta

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including EXONDYS 51, designed to skip exon 51 and approved under the accelerated approval pathway. For more information, please visit us at www.sarepta.com.

Contacts

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Sarepta Forward-looking Statements

This press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as “believes,” “anticipates,” “plans,” “expects,” “will,” “intends,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements about the terms of the license and collaboration agreement Sarepta has entered into with Summit (Oxford) LTD, including the rights, obligations and benefits of each party under the agreement such as Sarepta’s commercialization rights for certain product candidates in specified territories and Sarepta’s payments associated with those rights to Summit; the potential of ezutromid and utrophin modulation as a disease-modifying treatment for all patients with DMD regardless of their dystrophin gene mutation; the potential benefits to the parties and the DMD community resulting from the agreement; the partnership between the parties furthering their common goal of improving the lives of patients with DMD; the potential of utrophin modulation technology to complement Sarepta’s current approach of exon skipping therapy; Summit’s plans to access Sarepta’s expertise for the continued advancement of their promising utrophin modulator pipeline and working together to bring great advances to patients and families living with DMD.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta’s control. Known risk factors include, among others: the expected benefits and opportunities related to the license and collaboration and agreement may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the partnership between Sarepta and Summit may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreement, including any inability by Sarepta to fulfill its financial commitments to Summit; and even if the agreement results in commercialized products the parties may not achieve any significant revenues from the sale of such products.

Any of the foregoing risks could adversely affect Sarepta’s business, results of operations and the trading price of Sarepta’s common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta’s 2015 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended June 30, 2016 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

Summit Forward-looking Statements

Any statements in this press release about Summit’s future expectations, plans and prospects, including but not limited to, statements about the potential benefits and future operation of the collaboration with

Sarepta Therapeutics, including any potential future payments thereunder, clinical and preclinical development of Summit's product candidates, the therapeutic potential of Summit's product candidates, and the timing of initiation, completion and availability of data from clinical trials, and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the uncertainties inherent in the initiation of future clinical trials, availability and timing of data from ongoing and future clinical trials and the results of such trials, whether preliminary results from a clinical trial will be predictive of the final results of that trial or whether results of early clinical trials or preclinical studies will be indicative of the results of later clinical trials, expectations for regulatory approvals, availability of funding sufficient for Summit's foreseeable and unforeseeable operating expenses and capital expenditure requirements and other factors discussed in the "Risk Factors" section of filings that Summit makes with the Securities and Exchange Commission including Summit's Annual Report on Form 20-F for the fiscal year ended January 31, 2016. Accordingly readers should not place undue reliance on forward-looking statements or information. In addition, any forward-looking statements included in this press release represent Summit's views only as of the date of this release and should not be relied upon as representing Summit's views as of any subsequent date. Summit specifically disclaims any obligation to update any forward-looking statements included in this press release.

-END-

Exhibit 4.24

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

DATED 3rd MARCH 2017

(1) MUOX LIMITED

and

(2) OXFORD UNIVERSITY INNOVATION LIMITED
(FORMERLY ISIS INNOVATION LIMITED)

and

(3) SUMMIT (OXFORD) LIMITED

DEED OF NOVATION AND VARIATION

THIS DEED (the "**Deed**") is dated 3rd March 2017 ("the Completion Date")

BETWEEN:-

(1) **MUOX LIMITED**, a company incorporated in England (company number 08338316) whose registered office is at 9400 Garsington Road, Oxford Business Park, Oxford, England, OX4 2HN ("**MuOX**");

(2) **OXFORD UNIVERSITY INNOVATION LIMITED** (**formerly Isis Innovation Limited**), a company incorporated in England (company number 2199542), whose registered office is at University Offices, Wellington Square, Oxford OX1 2JD, England ("**OUI**"); and

(3) **SUMMIT** (**OXFORD**) **LIMITED**, a company incorporated in England (company number 04636431), whose registered office is at 85B Park Drive, Milton Park, Abingdon, OX14 4RY, England ("**Summit**"),

each a "**Party**" and together the "**Parties**".

BACKGROUND:-

(A) On 22 November 2013 MuOX and OUI entered into Deed of Licence of Know-How (hereinafter the "Deed of Licence").

(B) The Parties have agreed to assign the benefit and burden of the Deed of Licence from MuOX to Summit, a member of the same group of companies as MuOX, on the terms set forth in this Deed.

(C) Separately, the Parties have agreed to amend the Deed of Licence on the terms set forth on this Deed.

THIS DEED WITNESSES as follows:-

1. DEFINITIONS AND INTERPRETATION

1.1 Clause headings shall not affect the interpretation or construction of this Deed. References to Clauses and Schedules are to the Clauses and Schedules of this Deed.

1.2 Unless the context otherwise requires, words in the singular include the plural and in the plural include the singular and a reference to one gender shall include a reference to all other genders.

1.3 Reference to a person includes a natural person, corporate or unincorporated body (whether or not having separate legal personality) and that person's legal and personal representatives, successors and permitted assigns.

1.4 A reference to a statute, statutory provision or subordinated legislation is a reference to it as it is in force from time to time, or extended obligation, liability or restriction on, or

otherwise adversely affects the rights of, any Party. A reference to a statute or statutory provision shall include any subordinate legislation made from time to time under that statute or statutory provision.

1.5 Any words following the terms **including**, **include**, **in particular** or any similar expression shall be construed as illustrative and shall not limit the sense of the words preceding those terms.

2. NOVATION

2.1 With effect from the Completion Date and subject to Clause 5, MuOX transfers all its rights and obligations under the Deed of Licence to Summit. Summit shall enjoy all the rights and benefits of MuOX under the Deed of Licence, and all references to MuOX in the Deed of Licence shall be read and construed as references to Summit.

2.2 With effect from the Completion Date, Summit undertakes to each of MuOX and OUI to perform the Deed of Licence and be bound by its terms in every way as if Summit had been a party to it in place of MuOX.

2.3 OUI:

1.1.1 irrevocably and unconditionally consents to the novation of the Deed of Licence by the substitution of MuOX by Summit and expressly agrees that the Deed of Licence shall be deemed to have been made with Summit as from the Completion Date for all purposes and in respect of all rights, benefits and obligations hereunder; and

1.1.2 agrees to perform the Deed of Licence and be bound by its terms in every way as if Summit were the original party to it in place of MuOX.

3. RELEASE

3.1 Subject to Clause 5, OUI and MuOX hereby mutually release each other from their obligations under the Deed of Licence as from the Completion Date.

3.2 OUI releases and discharges MuOX from all claims and demands whatsoever in respect of the Deed of Licence arising after the Completion Date and accepts the liability of Summit under the Deed of Licence from the Completion Date. MuOX shall remain liable for all liabilities and obligations under the Deed of Licence arising prior to the Completion Date.

4. VARIATION

4.1 With effect from the Completion Date, Summit and OUI agree that the Deed of Licence be amended as outlined in Clause 4.2 and Clause 4.3.

4.2 Clause 2.2.4 of the Deed of Licence shall be deleted entirely and replaced with:

"*the licence granted under clause 2.1 in respect of the Project 4417 Know How shall automatically convert to a non-exclusive licence on the third anniversary of the Effective Date. The licence granted under clause 2.1 in respect of the Project 8066 Know How shall remain exclusive until expiration of the Sponsored Research Agreement between Summit Therapeutics PLC (formerly Summit Corporation PLC), OUI and the University of Oxford entered into on 22 November 2013 ("the SRA").*"

4.3 A new Clause 2.5 be inserted to the Deed of Licence as follows:

"2.5 In consideration for the exclusive licence to the Project 8066 Know How, Summit shall make the following payments to OUI:-

(a) [**] pounds sterling (£[**]) payable within seven (7) days of the Completion Date against an invoice issued by OUI to Summit on the Completion Date;

(b) [**] pounds Sterling (£[**]) payable on 22 November 2017 against an invoice issued by OUI to Summit on the same date;

(c) [**] pounds Sterling (£[**]) payable on 22 November 2018 against an invoice issued by OUI to Summit on the same date; and

(d) *in the event Summit Therapeutics PLC elects to exercise its option to extend the term of the SRA, [**] pounds Sterling (£[**]) will be payable on 22 November 2019 against an invoice issued by OUI to Summit on the same date.*

4.4 The first sentence of clause 10.5 of the Deed of Licence shall be deleted entirely and replaced with as follows:

"10.5 All notices to be sent to OUI under this agreement must indicate the OUI Project No. and should be sent, by post and fax unless agreed otherwise in writing, until further notice to: The Managing Director, Oxford University Innovation Ltd, Buxton Court, 3 West Way, Oxford OX2 OJB, Fax: +44 (0)1865 280831."

5. CONFIDENTIALITY

Notwithstanding anything else in this Deed, MuOX shall remain bound by the confidentiality provisions set out in the Deed of Licence and hereby agrees to be liable for any breach of those provisions by MuOX, its employees and/or agents to Summit and OUI.

6. GENERAL

6.1 Further Assurance

All Parties shall (at its own expense) promptly execute and deliver all such documents, and do all such things, or procure the execution of documents and doing of such things as are reasonably required to give full effect to this Deed and the transactions contemplated by it.

6.2 Severance

The invalidity or unenforceability of any provision of or any part of a provision of or any right arising pursuant to this Deed shall not affect in any way the remaining provisions or rights, which shall be construed as if such invalid or unenforceable part did not exist.

7. GOVERNING LAW

This Deed is governed by and shall be construed in accordance with English law.

8. JURISDICTION

The Parties hereby submit to the exclusive jurisdiction of the Royal Courts of Justice in London, England in relation to any dispute or claim arising out of, or in connection with, this Deed or in relation to its existence or validity (including non-contractual disputes or claims) which is not resolved by the applicable dispute resolution procedure hereunder.

This Deed has been executed as a deed and is delivered and takes effect on the Completion Date.

Executed as a deed by **MUOX LIMITED** acting by Glyn O. Edwards, a director in the presence of

/s/ Glyn Edwards
Director

Signature of witness: /s/ Melissa Strange

Name of witness: Melissa Strange

Address: [***]

Occupation: FCCA, Accountant

Executed as a deed by **SUMMIT (OXFORD) LIMITED** acting by Glyn O. Edwards, a director and

/s/ Glyn Edwards
Director

Melissa Strange

/s/ Melissa Strange
Secretary

Executed as a deed by **OXFORD UNIVERSITY INNOVATION LIMITED** acting by

/s/ Matthew Perkins
Director

A director and

/s/ L.A. Naylor
[Director/~~Secretary~~]

Exhibit 4.25

BROOK STREET DES ROCHES

DATED 17 FEBRUARY 2017

(1) MEPC MILTON PARK NO. 1 LIMITED AND MEPC MILTON PARK NO. 2 LIMITED

(2) SUMMIT THERAPEUTICS PLC

LEASE

---

relating to

136A Eastern Avenue

Milton Park

+44 (0) 1235 836600
BSDR.COM
DX 144160 ABINGDON 4

BrookStreet des Roches LLP
25A Western Avenue, Milton Park,
Abingdon, Oxfordshire, OX14 4SH

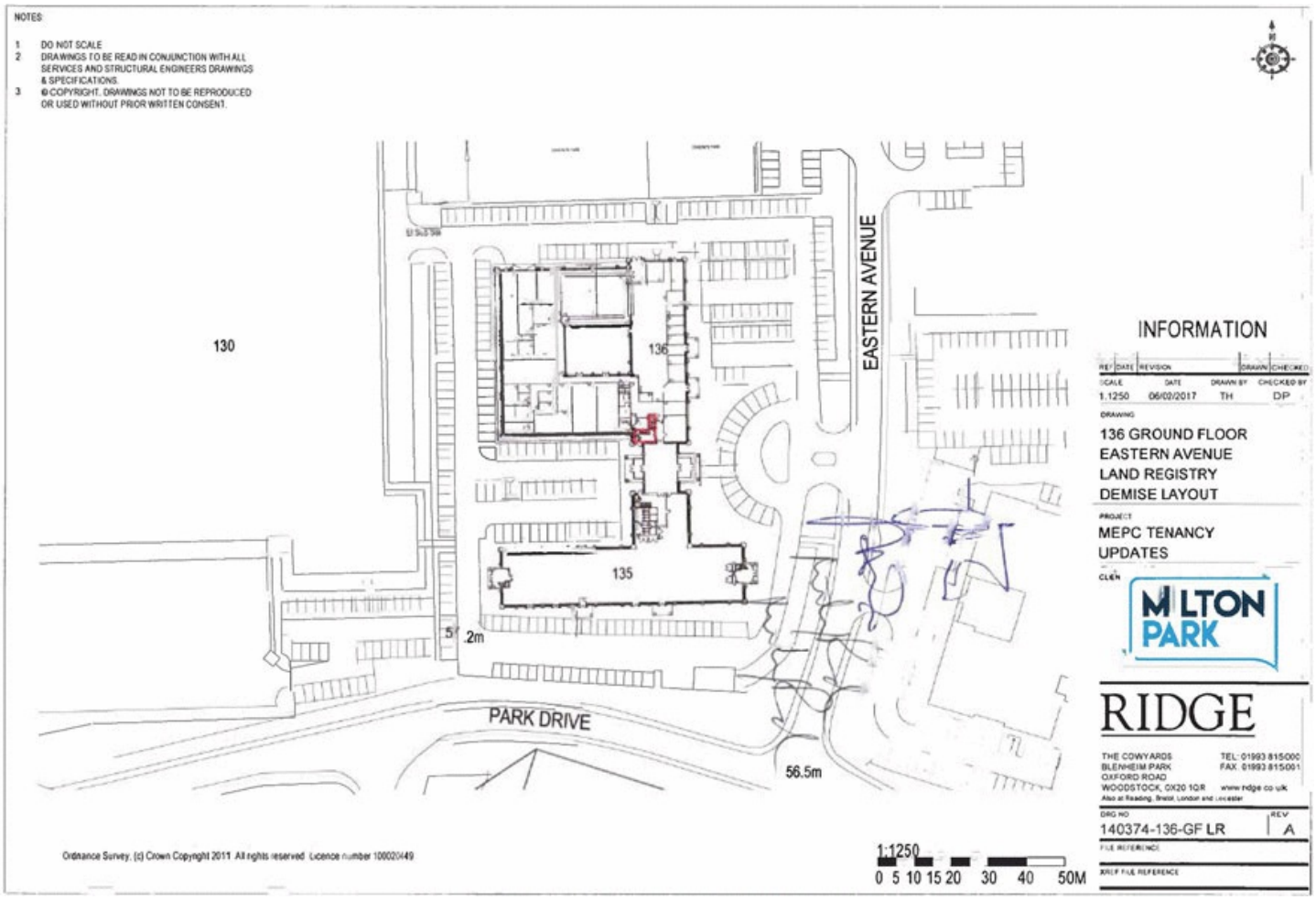
MEPC Milton Park

PRESCRIBED CLAUSES

LR1.	**Date of lease**	:	17 February 2017
LR2.	**Title number(s)**		**LR2.1 Landlord's title number(s)**
			BK102078
			LR2.2 Other title number(s)
			ON122118, ON122717, ON130606, ON145942, ON146219, ON225380, ON38283, ON72772, ON96949, ON216090
LR3.	**Parties to this lease**	:	**Landlord**
			MEPC MILTON PARK NO. 1 LIMITED (Company number 5491670) and **MEPC MILTON PARK NO. 2 LIMITED** (Company number 5491806), on behalf of MEPC Milton LP (LP No. LP14504), both of whose registered offices are at Lloyds Chambers 1 Portsoken Street London E1 8HZ
			Tenant
			SUMMIT THERAPEUTICS PLC (Company number 05197494) whose registered office is at 85b Park Drive Milton Park Abingdon Oxfordshire OX14 4RY
			Other parties
			None
LR4.	**Property**	:	**In the case of a conflict between this clause and the remainder of this lease then, for the purposes of registration, this clause shall prevail.**
			Ground and First floor premises known as 136A Eastern Avenue, Milton Park, Abingdon, Oxfordshire OX14 4SB shown edged red on Plans 1A and 1B with a net internal floor area of 622.0 square metres (6,781 square feet) measured in accordance with the RICS Code of Measuring Practice (sixth edition)
LR5.	**Prescribed Statements etc.**	:	None
LR6.	**Term for which the Property is leased**		From and including 17 February 2017
			To and including 16 February 2027
LR7.	**Premium**		None
LR8.	**Prohibitions or restrictions on disposing of this lease**		This lease contains a provision that prohibits or restricts dispositions
LR9.	**Rights of acquisition etc.**		**LR9.1 Tenant's contractual rights to renew this lease, to acquire the reversion or another lease of the Property, or to acquire an interest in other land**
			None
			LR9.2 Tenant's covenant to (or offer to) surrender this lease
			None
			LR9.3 Landlord's contractual rights to acquire this lease
			None

LR10.	**Restrictive covenants given in this lease by the Landlord in respect of land other than the Property**	None
LR11.	**Easements**	**LR11.1 Easements granted by this lease for the benefit of the Property** The easements specified in Part I of the First Schedule of this lease **LR11.2 Easements granted or reserved by this lease over the Property for the benefit of other property** The easements specified in Part II of the First Schedule of this lease
LR12.	**Estate rentcharge burdening the Property**	None
LR13.	**Application for standard form of restriction**	None
LR14.	**Declaration of trust where there is more than one person comprising the Tenant**	None

Plan 1A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MILTON PARK
RIDGE
THE COWYARDS
BLENHEIM PARK
OXFORD ROAD
WOODSTOCK, OX20 1QR
TEL: 01993 815000
FAX: 01993 815001
www.ridge.co.uk
Also at Reading, Bristol, London and Leicester
DRG NO
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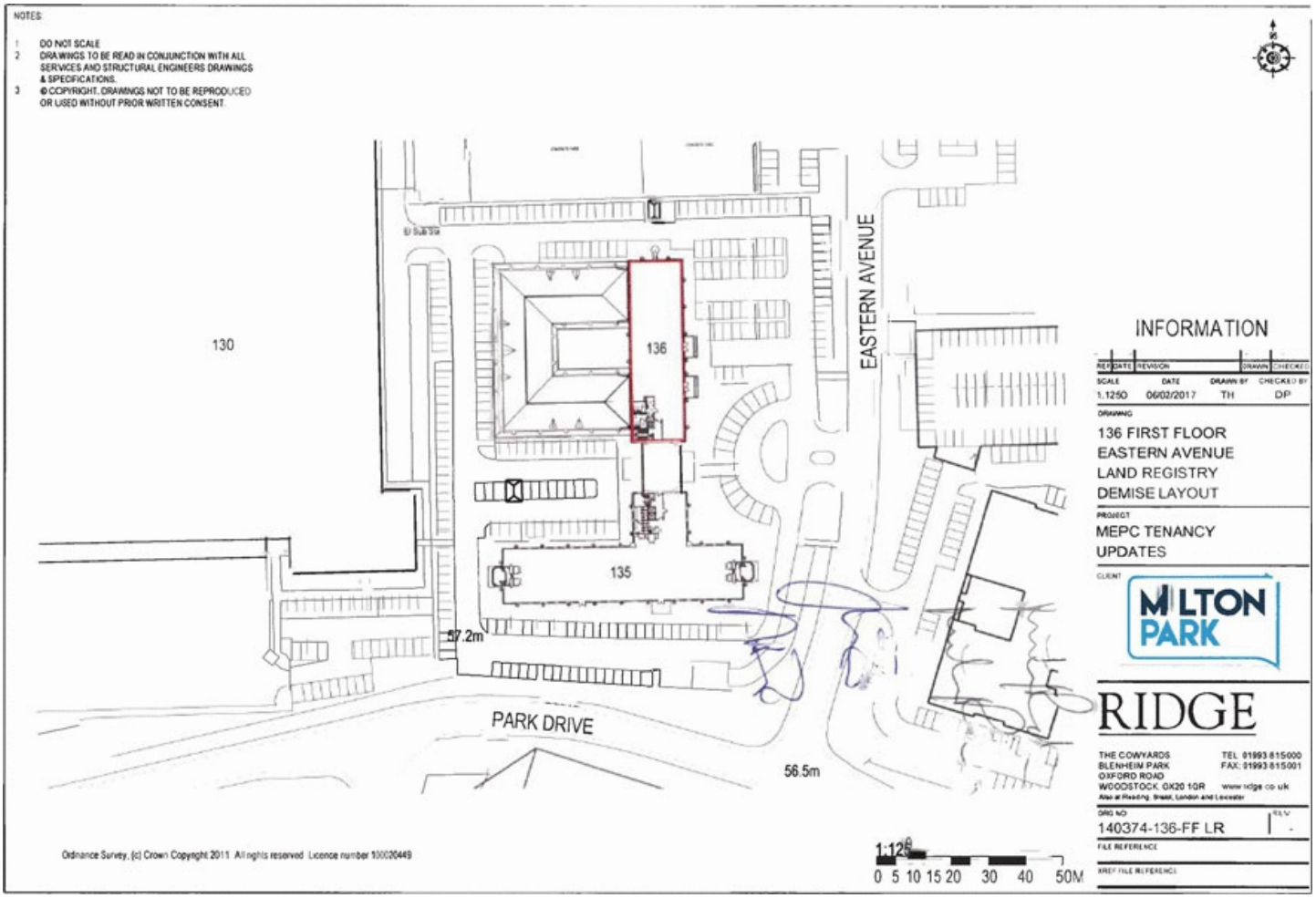
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OXFORD ROAD
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TEL 01993 815000
FAX: 01993 815001
www.ridge.co.uk
Also at Reading, Bristol, London and Leicester
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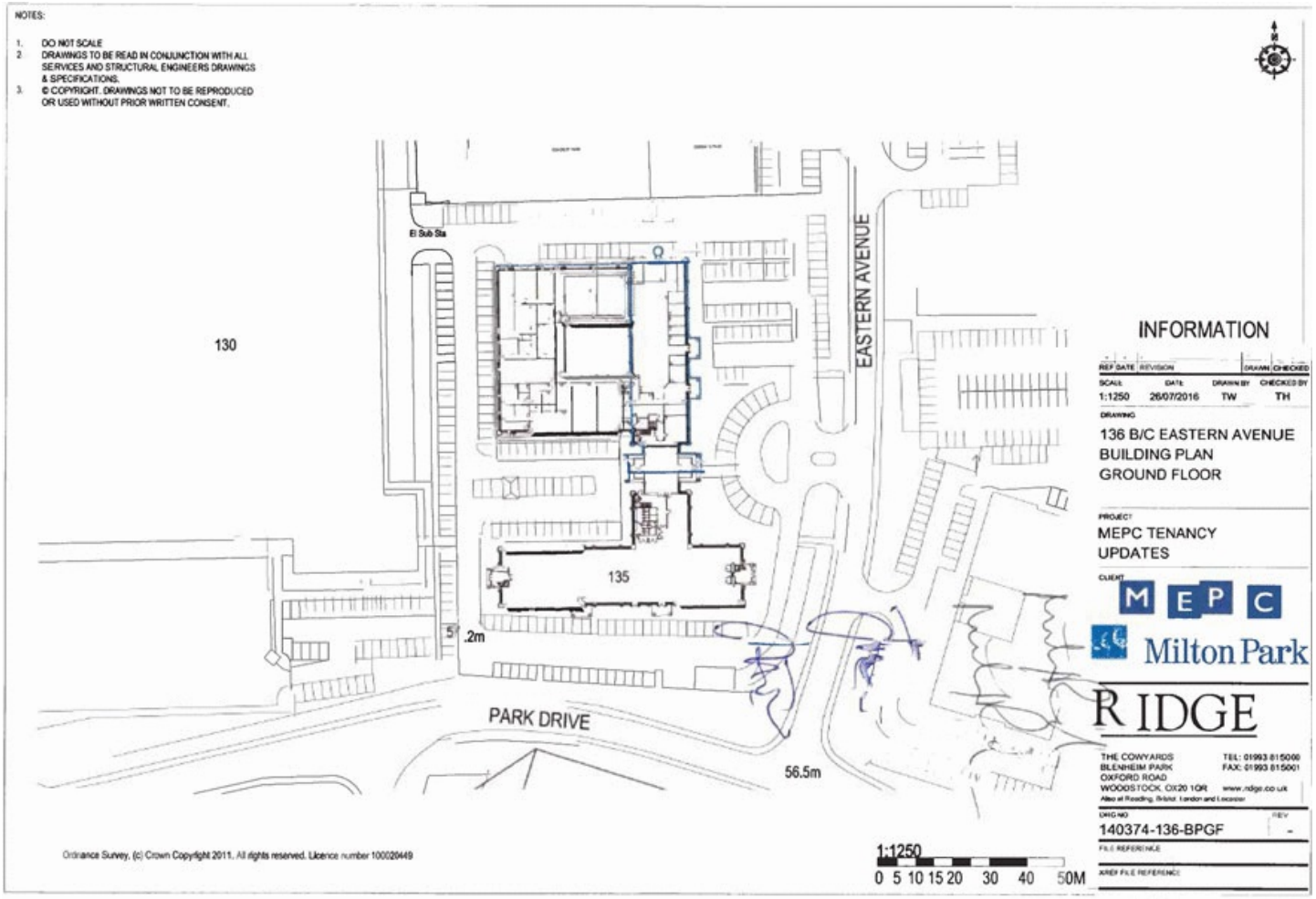
Plan 2A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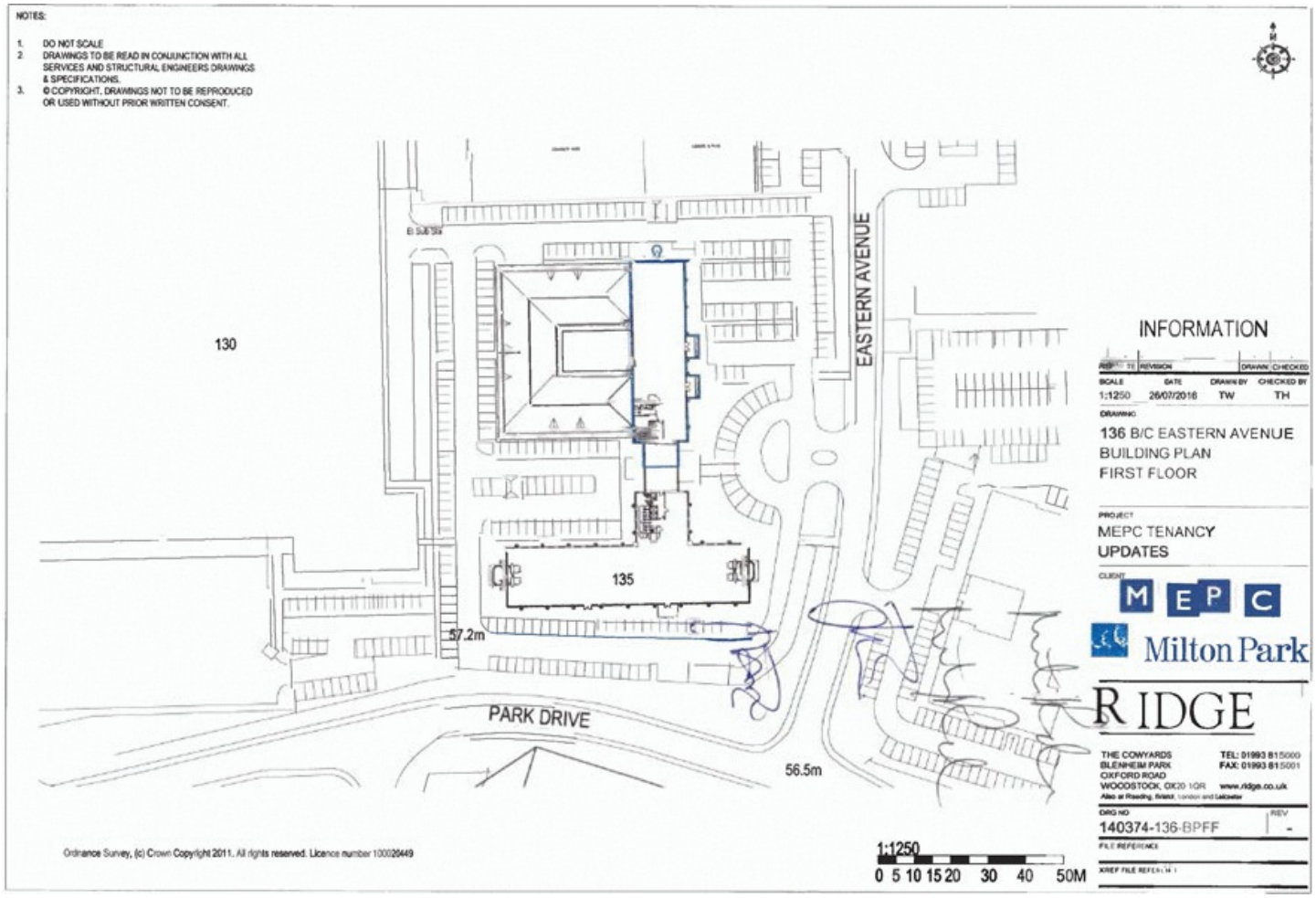
Plan 2B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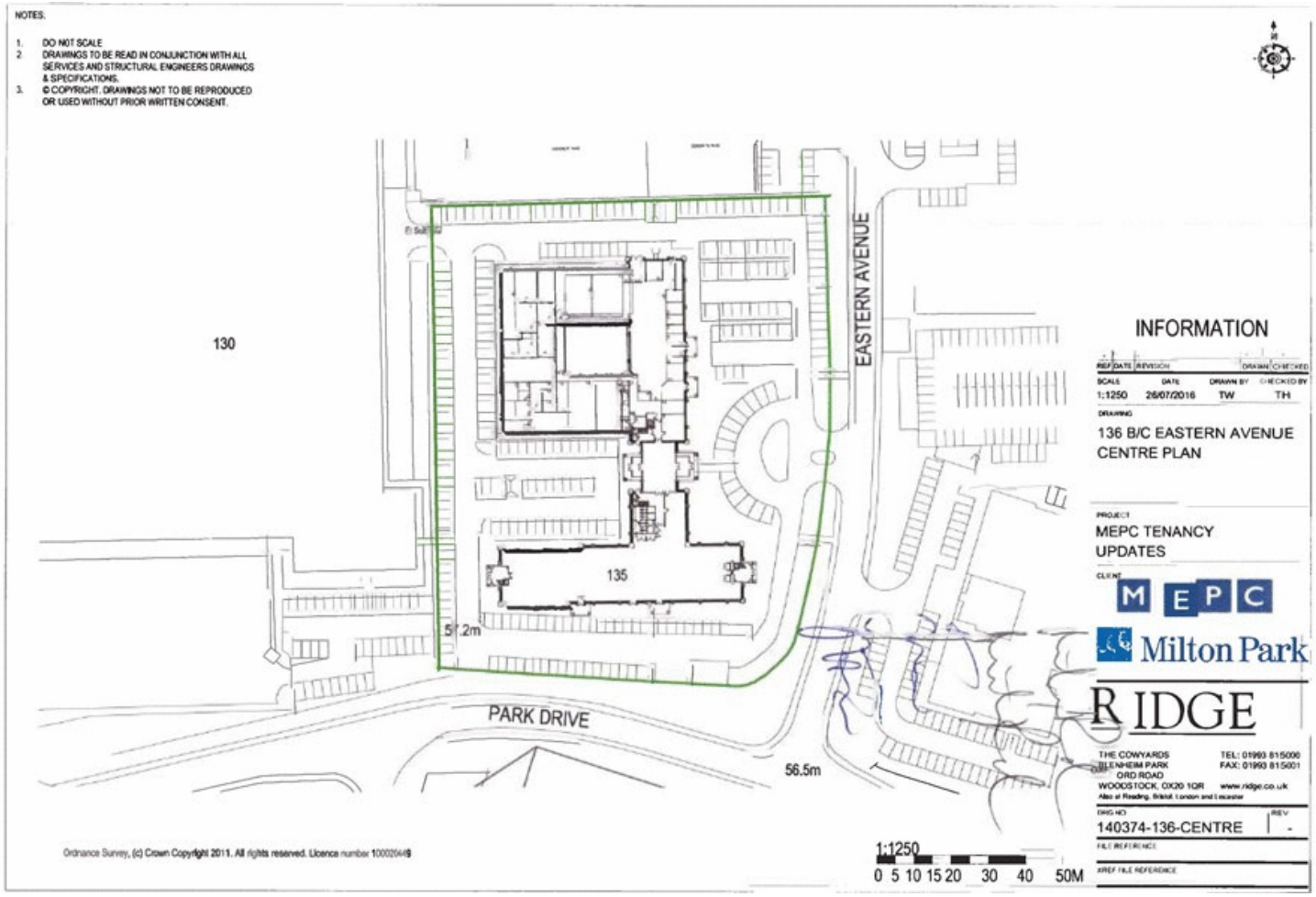
Plan 3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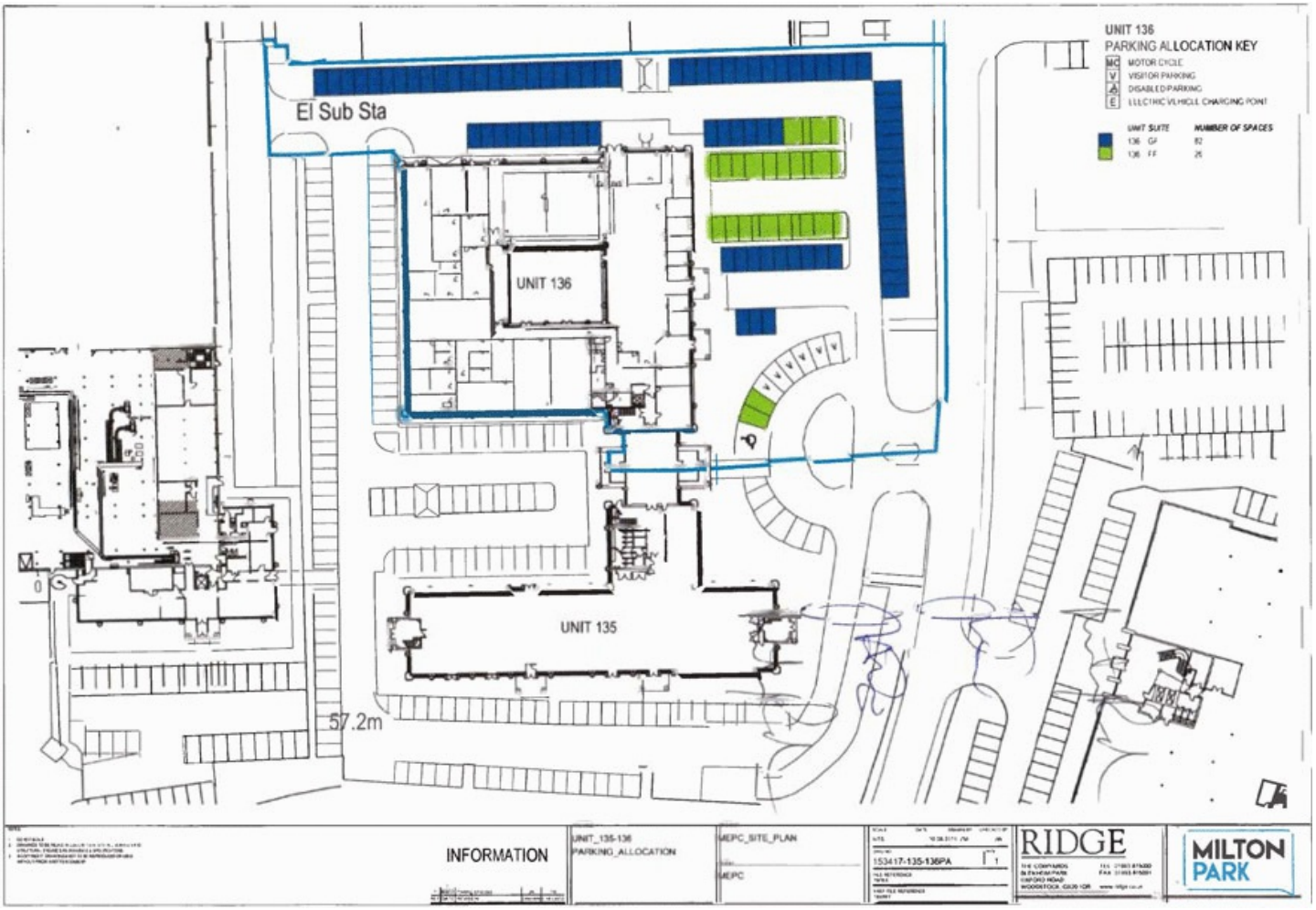
UNIT 136
PARKING ALLOCATION KEY
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V VISITOR PARKING
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PARKING_ALLOCATION
MEPC_SITE_PLAN
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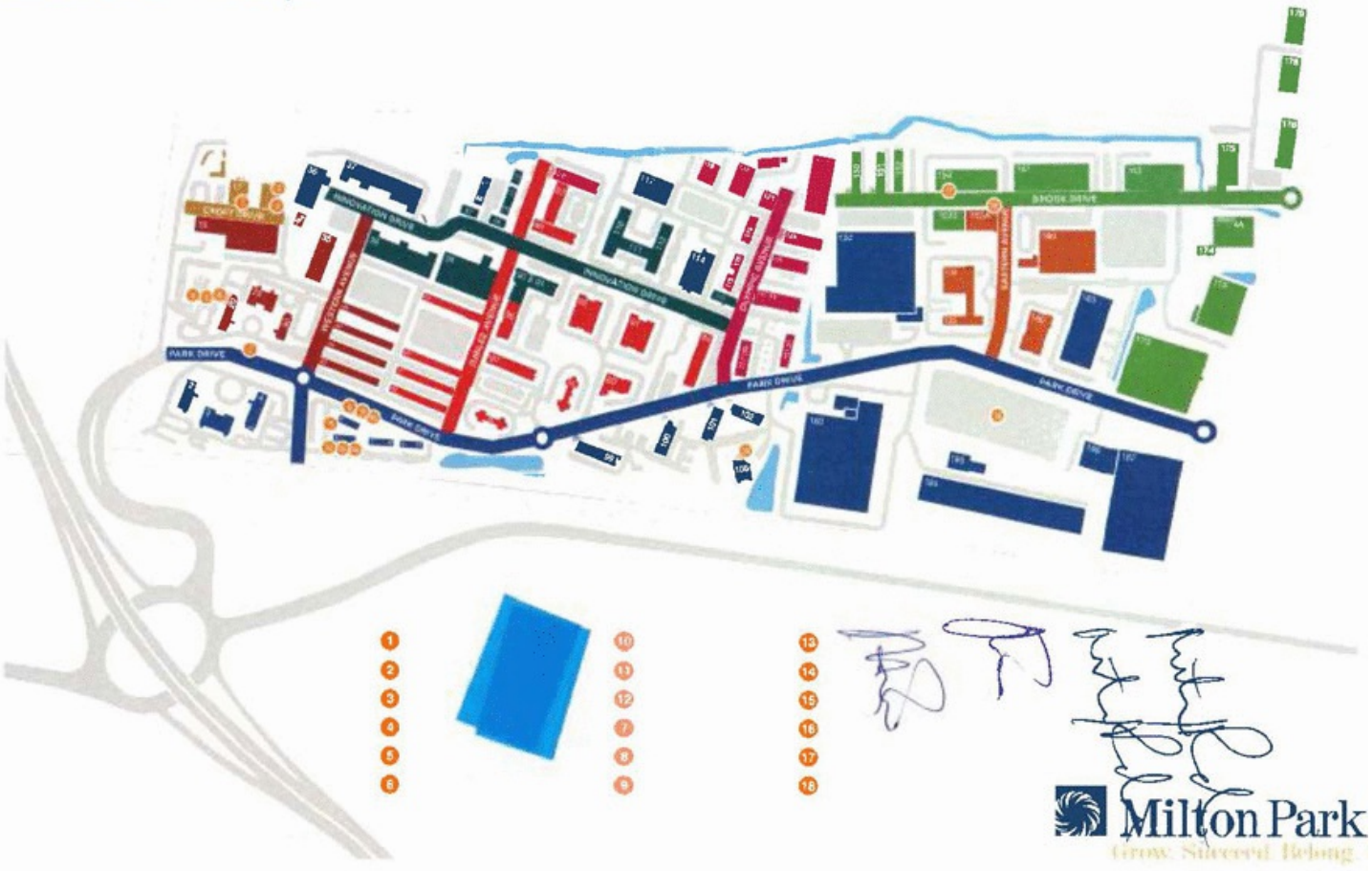
RIDGE
MILTON
PARK

Estate Map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Milton Park

This lease made on the date and between the parties specified in the Prescribed Clauses **Witnesses** as follows:

1 Definitions and Interpretation

In this lease unless the context otherwise requires:

1.1 Definitions

Adjoining Property means any adjoining or neighbouring premises in which the Landlord or a Group Company of the Landlord holds or shall at any time during the Term hold a freehold or leasehold interest;

Base Rate means the base rate from time to time of Barclays Bank PLC or (if not available) such comparable rate of interest as the Landlord shall reasonably require;

Break Date means 17 February 2022;

Break Payment means FIFTY FIVE THOUSAND THREE HUNDRED AND SEVENTY EIGHT POUNDS (£55,378);

Building means that part of the Centre which comprises a two-storey building (of which the Property forms part) and shown for the purposes of identification edged blue on Plans 2A and 2B and includes any part of it and any alteration or addition to it or replacement of it;

Building Services means the services provided or procured by the Landlord in relation to the Building as set out in Part IV of the Fourth Schedule;

Common Parts means the accesses, and other areas of the Building from time to time designated by the Landlord for common use by the tenants and occupiers of the Building;

Centre means the part of the Estate shown edged green on Plan 3 (of which the Building forms part) and includes any part of it and any alteration or addition to it or replacement of it and any additional buildings constructed on it;

Centre Common Areas means the roads, accesses, the parking and other areas of the Centre from time to time designated by the Landlord for common use by the tenants and occupiers of the Centre;

Centre Services means the services provided or procured by the Landlord in relation to the Centre as set out in Part III of the Fourth Schedule;

Concessionary Rent Period means the period beginning on the date on which the Contractual Term begins and expiring on and including 16 February 2019;

Conduit means any existing or future media for the passage of substances or energy and any ancillary apparatus attached to them and any enclosures for them;

Contractual Term means the term specified in the Prescribed Clauses;

Encumbrances means the obligations and encumbrances (if any) specified in Part III of the First Schedule;

Estate means Milton Park, Abingdon, Oxfordshire (of which the Centre forms part) and the buildings from time to time standing on it shown on the Plan together with any other adjoining land which is incorporated into Milton Park;

Estate Common Areas means the roads, accesses, landscaped areas, car parks, estate management offices and other areas or amenities on the Estate or outside the Estate but serving or otherwise benefiting the Estate as a whole which are from time to time provided or designated for the common amenity or benefit of the owners or occupiers of the Estate;

Estate Services means the services provided or procured by the Landlord in relation to the Estate as set out in Part II of the Fourth Schedule;

Group Company means a company which is a member of the same group of companies within the meaning of Section 42 of the 1954 Act;

Guarantor means any party to this lease so named in the Prescribed Clauses (which in the case of an individual includes his personal representatives) and any guarantor of the obligations of the Tenant for the time being;

Insurance Commencement Date means 17 February 2017;

Insured Risks means fire, lightning, earthquake, explosion, aircraft (other than hostile aircraft) and other aerial devices or articles dropped therefrom, riot, civil commotion, malicious damage, terrorism, storm or tempest, bursting or overflowing of water tanks apparatus or pipes, flood and impact by road vehicles, subsidence, landslip and heave (to the extent that insurance against such risks may ordinarily be arranged with an insurer of good repute) and such other risks or insurance as may from time to time be reasonably required by the Landlord (subject in all cases to such usual exclusions and limitations as may be imposed by the insurers), and **Insured Risk** means any one of them;

Landlord means the party to this lease so named in the Prescribed Clauses and includes any other person entitled to the immediate reversion to this lease;

Landlord's Surveyor means a suitably qualified person or firm appointed by the Landlord (including an employee of the Landlord or a Group Company) to perform the function of a surveyor for the purposes of this lease;

Lease Particulars means the descriptions and terms in the section headed **Lease Particulars** which form part of this lease insofar as they are not inconsistent with the other provisions of this lease;

Lettable Units means any part of the Building which is let or constructed or adapted for letting from time to time;

Permitted Use means use as offices within Class B1(a) of the 1987 Order;

Plan means the plan or plans annexed to this lease;

Prescribed Clauses means the descriptions and terms in the section headed **Prescribed Clauses** which form part of this lease;

Principal Rent means:

From and including the Rent Commencement Date to and including 16 February 2018: THIRTY TWO THOUSAND EIGHT HUNDRED AND EIGHTY EIGHT POUNDS (£32,888) per annum;

From and including 17 February 2018 to and including 16 February 2019: ONE HUNDRED AND THIRTY TWO THOUSAND NINE HUNDRED AND EIGHT POUNDS (£132,908) per annum;

From and including 17 February 2019 to and including 16 February 2022: ONE HUNDRED AND SIXTY SIX THOUSAND ONE HUNDRED AND THIRTY FIVE POUNDS (£166,135) per annum

subject to increase in accordance with the Second Schedule;

Property means the property described in the Prescribed Clauses and includes any part of it any alteration or addition to the Property and any fixtures and fittings in or on the Property and includes:-

(i) the floorboards, screed, plaster and other finishes on the floors, walls, columns and ceilings, and all carpets;

(ii) the raised floors and false ceilings (including light fittings) and the voids between the ceilings and false ceilings and the floor slab and the raised floors;

(iii) non-load bearing walls and columns in the Property and one half of the thickness of such walls dividing the Property from other parts of the Building;

(iv) all doors and internal windows and their frames, glass and fitments;

(v) all Conduits, plant and machinery within and solely serving the same;

(vi) all Landlord's fixtures and fittings;

(vii) all alterations and additions;

but excludes:

(i) all structural and external parts of the Building;

(ii) all Conduits, plant and machinery serving other parts of the Building;

Quarter Days means 25 March, 24 June, 29 September and 25 December in every year and **Quarter Day** means any of them;

Rent Commencement Date means 17 February 2017;

Review Date means 17 February 2022;

Service Charge means the Service Charge set out in the Fourth Schedule;

Service Charge Commencement Date means 17 February 2017;

Services means the Estate Services, the Centre Services and the Building Services;

Tenant means the party to this lease so named in the Prescribed Clauses and includes its successors in title;

Term means the Contractual Term together with any continuation of the term or the tenancy (whether by statute, common law holding over or otherwise);

This lease means this lease and any document supplemental to it or entered into pursuant to it;

Uninsured Risk means an Insured Risk against which insurance is from time to time unobtainable on normal commercial terms in the London insurance market at reasonable commercial rates for a property equivalent in size, layout, type and location;

VAT means Value Added Tax and any similar tax substituted for it or levied in addition to it;

1954 Act means the Landlord and Tenant Act 1954;

1987 Order means the Town and Country Planning (Use Classes) Order 1987 (as originally made);

1995 Act means the Landlord and Tenant (Covenants) Act 1995;

2003 Order means The Regulatory Reform (Business Tenancies) (England and Wales) Order 2003.

1.2 Interpretation

1.2.1 If the Tenant or the Guarantor is more than one person then their covenants are joint and several;

1.2.2 Any reference to a statute includes any modification extension or re-enactment of it and any orders, regulations, directions, schemes and rules made under it;

1.2.3 Any covenant by the Tenant not to do any act or thing includes an obligation not knowingly to permit or suffer such act or thing to be done;

1.2.4 If the Landlord reserves rights of access or other rights over or in relation to the Property then those rights extend to persons authorised by it;

1.2.5 References to the **act or default of the Tenant** include acts or default or negligence of any undertenant or of anyone at the Property with the Tenant's or any undertenant's permission or sufferance;

1.2.6 The index and Clause headings in this lease are for ease of reference only;

1.2.7 References to the **last year of the Term** shall mean the twelve months ending on the expiration or earlier termination of the Term;

1.2.8 References to **Costs** include all liabilities, claims, demands, proceedings, damages, losses and proper and reasonable costs and expenses;

1.2.9 References to Principal Rent and Revised Rent are references to yearly sums.

2 Demise

The Landlord with Full Title Guarantee DEMISES the Property to the Tenant for the Contractual Term TOGETHER WITH the rights set out in Part I of the First Schedule, EXCEPT AND RESERVING as mentioned in Part II of the First Schedule and SUBJECT TO the Encumbrances;

3 Rent

The Tenant will pay by way of rent during the Term or until released pursuant to the 1995 Act without any deduction counterclaim or set off except where required by law:

3.1 The Principal Rent and any VAT by equal quarterly payments in advance on the Quarter Days to be paid by Direct Debit, Banker's Standing Order or other means as the Landlord reasonably requires, the first payment for the period from and including the Rent Commencement Date to (but excluding) the next Quarter Day to be made on the Rent Commencement Date;

3.2 The Service Charge and any VAT at the times and in the manner set out in the Fourth Schedule;

3.3 The following amounts and any VAT:

3.3.1 the sums specified in Clauses 4.1 [interest] and 4.2 [outgoings and utilities];

3.3.2 the sums specified in Clause 6.2.1 [insurance];

3.3.3 all Costs incurred by the Landlord as a result of any breach of the Tenant's covenants in this lease.

4 Tenant's covenants

The Tenant covenants with the Landlord throughout the Term, or until released pursuant to the 1995 Act, as follows:

4.1 Interest

If the Landlord does not receive any sum due to it within 14 days of the due date to pay on demand interest on such sum at 2 per cent above Base Rate from the due date until payment (both before and after any judgment), provided this Clause shall not prejudice any other right or remedy for the recovery of such sum;

4.2 Outgoings and Utilities

4.2.1 To pay all existing and future rates, taxes, charges, assessments and outgoings in respect of the Property (whether assessed or imposed on the owner or the occupier), except any tax (other than VAT) arising as a result of the receipt by the Landlord of the rents reserved by this lease and any tax arising on any dealing by the Landlord with its reversion to this lease;

4.2.2 To pay for all gas, electricity, water, telephone and other utilities used on the Property, and all charges in connection with such utilities and for meters and all standing charges, and a fair and reasonable proportion of any joint charges as determined by the Landlord's Surveyor;

4.3 VAT

4.3.1 Any payment or other consideration to be provided to the Landlord is exclusive of VAT, and the Tenant shall in addition pay any VAT chargeable on the date the payment or other consideration is due;

4.3.2 Any obligation to reimburse or pay the Landlord's expenditure extends to irrecoverable VAT on that expenditure, and the Tenant shall also reimburse or pay such VAT;

4.4 Repair

4.4.1 To keep the Property and any Conduits plant and equipment serving only the Property in good and substantial repair and condition (damage by the Insured Risks excepted save to the extent that insurance moneys are irrecoverable as a result of the act or default of the Tenant and damage by an Uninsured Risk also excepted);

4.4.2 To make good any disrepair for which the Tenant is liable within 2 months after the date of written notice from the Landlord (or sooner if the Landlord reasonably requires);

4.4.3 If the Tenant fails to comply with any such notice the Landlord may enter and carry out the work and the cost shall be reimbursed by the Tenant on demand as a debt;

4.4.4 To enter into maintenance contracts with reputable contractors for the regular servicing of all plant and equipment serving only the Property;

4.5 Decoration

4.5.1 To clean, prepare and paint or treat and generally redecorate all internal parts of the Property in every fifth year and, if different, in the last year of the Term;

4.5.2 All the work described in Clause 4.5.1 is to be carried out:

(i) in a good and workmanlike manner to the Landlord's reasonable satisfaction; and

(ii) in colours which (if different from the existing colour) are first approved in writing by the Landlord (approval not to be unreasonably withheld or delayed);

4.6 Cleaning

4.6.1 To keep the Property clean, tidy and free from rubbish;

4.6.2 To clean the inside of windows and any washable surfaces at the Property as often as reasonably necessary;

4.7 Overloading

Not to overload the floors, ceilings or structure of the Property or the structure of the Building or any plant machinery or electrical installation serving the Property or the Building or the Centre;

4.8 Conduits

To keep the Conduits in or serving the Property clear and free from any noxious, harmful or deleterious substance, and to remove any obstruction and repair any damage to the Conduits as soon as reasonably practicable to the Landlord's reasonable satisfaction;

4.9 User

4.9.1 Not to use the Property otherwise than for the Permitted Use;

4.9.2 Not to use the Property for any purpose which is:

(i) noisy, offensive, dangerous, illegal, immoral or an actionable nuisance; or

(ii) which in the reasonable opinion of the Landlord causes damage or disturbance to the Landlord, or to owners or occupiers of any neighbouring property; or

(iii) which involves any substance which may be harmful, polluting or contaminating other than in quantities which are normal for and used in connection with the Permitted Use;

4.10 Signs

Not to erect any sign, notice or advertisement which is visible outside the Property without the Landlord's prior written consent;

4.11 Alterations

4.11.1 Not to make any alterations or additions which:

(i) affect the structure of the Building (including without limitation the roofs and foundations and the principal or load-bearing walls, floors, beams and columns);

(ii) merge the Property with any adjoining premises;

(iii) affect the external appearance of the Property;

(iv) affect the heating air-conditioning and ventilation systems at the Building;

4.11.2 Not to make any other alterations or additions to the Property without the Landlord's written consent (which is not to be unreasonably withheld or delayed, but is not required in the case of internal non-load bearing demountable partitioning provided plans showing the extent of such works are deposited with the Landlord promptly on completion of the works);

4.12 Preservation of Easements

4.12.1 Not to prejudice the acquisition of any right of light for the benefit of the Property and to preserve all rights of light and other easements enjoyed by the Property;

4.12.2 Promptly to give the Landlord notice if any easement enjoyed by the Property is obstructed, or any new easement affecting the Property is made or attempted;

4.13 Alienation

4.13.1 Not to:

(i) assign, charge, underlet or part with possession of the whole or part only of the Property nor to agree to do so except by an assignment or underletting of the whole of the Property permitted by this Clause 4.13;

(ii) share the possession or occupation of the whole or any part of the Property;

(iii) assign, part with or share any of the benefits or burdens of this lease, or any interest derived from it by a virtual assignment or other similar arrangement.

4.13.2 Assignment

Not to assign or agree to assign the whole of the Property without the Landlord's written consent (not to be unreasonably withheld or delayed), provided that:

(i) the Landlord may withhold consent in circumstances where in the reasonable opinion of the Landlord

(a) the proposed assignee is not of sufficient financial standing to enable it to comply with the Tenant's covenants in this lease; or

(b) such person as the Landlord reasonably requires does not act as guarantor for the assignee and does not enter into direct covenants with the Landlord including the provisions set out in the Third Schedule (but referring in paragraph 1.2 to the assignee);

(ii) the Landlord's consent shall in every case be subject to conditions (unless expressly excluded) requiring that:

(a) the assignee covenants with the Landlord to pay the rents and observe and perform the Tenant's covenants in this lease during the residue of the Term, or until released pursuant to the 1995 Act;

(b) the Tenant enters into an authorised guarantee agreement guaranteeing the performance of the Tenant's covenants in this lease by the assignee including the provisions set out in paragraphs 1-5 (inclusive) of the Third Schedule (but omitting paragraph 1.2);

(c) all rent and other payments due under this lease and demanded in writing not less than 14 days' prior to the assignment (not the subject of a bona fide dispute) are paid before completion of the assignment;

4.13.3 Underletting

Not to underlet or agree to underlet the whole of the Property nor vary the terms of any underlease without the Landlord's written consent (not to be unreasonably withheld or delayed). Any permitted underletting must comply with the following:

(i) the rent payable under the underlease must be:

(a) not less than the rent reasonably obtainable in the open market for the Property without fine or premium;

(b) payable no more than one quarter in advance;

(c) subject to upward only reviews at intervals no less frequent than the rent reviews under this lease;

(ii) the undertenant covenants with the Landlord and in the underlease:

(a) not to do anything which might amount to a breach of the Tenant's covenants in this lease during the term of the underlease or until released pursuant to the 1995 Act;

(b) to observe and perform the covenants on the part of the undertenant in the underlease during the term of the underlease or until released pursuant to the 1995 Act;

(c) not to underlet, share or part with possession or occupation of the whole or any part of the underlet premises, nor to assign or charge part only of the underlet premises;

(d) not to assign the whole of the underlet premises without the Landlord's prior written consent (which shall not be unreasonably withheld or delayed);

(iii) all rents and other payments due under this lease (not the subject of a bona fide dispute) are paid before completion of the underletting;

(iv) Sections 24 to 28 of the 1954 Act must be excluded and before completion of the underletting a certified copy of each of the following documents must be supplied to the Landlord:

(a) the notice served on the proposed undertenant pursuant to section 38A(3)(a) of the 1954 Act; and

(b) the declaration actually made by the proposed undertenant in compliance with the requirements of Schedule 2 of the 2003 Order; and

(c) the proposed form of underlease containing an agreement to exclude the provisions of sections 24 to 28 of the 1954 Act and a reference to both the notice pursuant to section 38A(3)(a) of the 1954 Act and the declaration pursuant to the requirements of Schedule 2 of the 2003 Order as referred to in this clause 4.13.3;

4.13.4 To take all necessary steps and proceedings to remedy any breach of the covenants of the undertenant under the underlease and not to permit any reduction of the rent payable by any undertenant other than owing to rent suspension pursuant to clause 6.3 of this lease incorporated by reference into the underlease;

4.13.5 Group Sharing

Notwithstanding Clause 4.13.1 the Tenant may share occupation of the whole or any part of the Property with a Group Company

PROVIDED THAT

(a) the relationship of landlord and tenant is not created; and

(b) occupation by any Group Company shall cease upon it ceasing to be a Group Company; and

(c) the Tenant informs the Landlord in writing before each occupier commences occupation and after it ceases occupation;

4.14 Registration

Within 21 days to give to the Landlord's solicitors (or as the Landlord may direct) written notice of any assignment, charge, underlease or other devolution of the Property together with a certified copy of the relevant document and a reasonable registration fee of not more than £50;

4.15 Statutory Requirements and Notices

4.15.1 To supply the Landlord with a copy of any notice, order or certificate or proposal for any notice order or certificate affecting or capable of affecting the Property as soon as it is received by or comes to the notice of the Tenant;

4.15.2 To comply promptly with all notices served by any public, local or statutory authority, and with the requirements of any present or future statute or European Union law, regulation or directive (whether imposed on the owner or occupier), which affects the Property or its use;

4.15.3 At the request of the Landlord, but at the joint cost of the Landlord and the Tenant**,** to make or join the Landlord in making such objections or representations against or in respect of any such notice, order or certificate as the Landlord may reasonably require;

4.16 Planning

4.16.1 Not without the consent of the Landlord (such consent not to be unreasonably withheld or delayed) to apply for or implement any planning permission affecting the Property (other than any permission for the Permitted Use);

4.16.2 If a planning permission is implemented the Tenant shall complete all the works permitted and comply with all the conditions imposed by the permission before the determination of the Term (including any works stipulated to be carried out by a date after the determination of the Term unless the Landlord requires otherwise);

4.17 Contaminants and Defects

4.17.1 To give the Landlord prompt written notice upon becoming aware of the existence of any defect in the Property, or of the existence of any contaminant, pollutant or harmful substance on the Property but not used in the ordinary course of the Tenant's use of the Property;

4.17.2 If so requested by the Landlord, to remove from the Property or remedy to the Landlord's reasonable satisfaction any such contaminant, pollutant or harmful substance introduced on the Property by or at the request of the Tenant;

4.18 Entry by Landlord

To permit the Landlord at all reasonable times and on reasonable notice (except in emergency) to enter the Property in order to:

4.18.1 inspect and record the condition of the Property or other parts of the Building or the Centre or the Adjoining Property;

4.18.2 remedy any breach of the Tenant's obligations under this lease;

4.18.3 repair, maintain, clean, alter, replace, install, add to or connect up to any Conduits or plant and machinery which serve the Building or the Centre or the Adjoining Property;

4.18.4 repair, maintain, alter or rebuild the Building or the Centre or the Adjoining Property;

4.18.5 comply with any of its obligations under this lease;

Provided that the Landlord shall cause as little inconvenience as reasonably practicable in the exercise of such rights and shall promptly make good all physical damage to the Property caused by such entry;

4.19 Landlord's Costs

To pay to the Landlord on demand amounts equal to such Costs as it may properly and reasonably incur:

4.19.1 in connection with any application for consent made necessary by this lease (including where consent is lawfully refused or the application is withdrawn);

4.19.2 incidental to or in reasonable contemplation of the preparation and service of a schedule of dilapidations (whether before or within three (3) months after the end of the Term) or a notice or proceedings under Section 146 or Section 147 of the Law of Property Act 1925 (even if forfeiture is avoided other than by relief granted by the Court);

4.19.3 in connection with the enforcement or remedying of any breach of the covenants in this lease on the part of the Tenant and any Guarantor;

4.19.4 incidental to or in reasonable contemplation of the preparation and service of any notice under Section 17 of the 1995 Act;

4.20 Yielding up

Immediately before the end of the Term:

(i) to give up the Property repaired and decorated and otherwise in accordance with the Tenant's covenants in this lease;

(ii) if the Landlord so requires to remove all alterations made during the Term or any preceding period of occupation by the Tenant and reinstate the Property as the Landlord shall reasonably direct and to its reasonable satisfaction;

(iii) to remove all signs, tenant's fixtures and fittings and other goods from the Property, and make good any damage caused thereby to the Landlord's reasonable satisfaction;

(iv) to replace any damaged or missing Landlord's fixtures with ones of no less quality and value;

(v) to replace all carpets with ones of no less quality and value than those in the Property at the start of the Contractual Term;

(vi) to give to the Landlord all operating and maintenance manuals together with any health and safety files relating to the Property;

(vii) to provide evidence of satisfactory condition and maintenance of plant and machinery including (without limitation) electrical installation condition reports in respect of all of the electrical circuits and supply equipment in the Property, other condition reports as required under any relevant statute or European Union law, regulation or directive and copies of all service records;

(viii) to return any security cards or passes (if any) provided by the Landlord for use by the Tenant and its visitors.

4.21 Encumbrances

To perform and observe the Encumbrances so far as they relate to the Property.

4.22 Roads Etc

Not to obstruct the roads, pavements, footpaths and forecourt areas from time to time on the Estate in any way whatsoever and not to use any part of the forecourts and car parking spaces or other open parts of the Property for the purpose of storage or deposit of any materials, goods, container ships' pallets, refuse, waste scrap or any other material or matter.

4.23 Parking Restrictions

Except as to any right specifically granted in this lease not to permit any vehicles belonging to or calling upon the Tenant to stand on the roads, car parking spaces, forecourts, pavements or footpaths on the Estate.

4.24 Regulations etc

4.24.1 At all times during the Term to observe and perform such regulations (if any) in respect of the Building or the Centre or the Estate as the Landlord may reasonably think expedient to the proper management of the Building or the Centre or the Estate and which are notified to the Tenant;

4.24.2 Not to cause any obstruction to the Common Parts or any part of the Building or the Centre or the Estate.

4.25 Land Registration Provisions

4.25.1 Promptly following the grant of this lease the Tenant shall apply to register this lease at the Land Registry and shall ensure that any requisitions raised by the Land Registry in connection with that application are dealt with promptly and properly and within one month after completion of the registration, the Tenant shall send the Landlord official copies of its title;

4.25.2 Immediately after the end of the Term (and notwithstanding that the Term has ended), the Tenant shall make an application to close the registered title of this lease and shall ensure that any requisitions raised by the Land Registry in connection with that application are dealt with promptly and properly and the Tenant shall keep the Landlord informed of the progress and completion of its application.

5 Landlord's Covenants

5.1 Quiet Enjoyment

The Landlord covenants with the Tenant that, the Tenant may peaceably enjoy the Property during the Term without any interruption by the Landlord or any person lawfully claiming under or in trust for it.

5.2 Provision of Services

The Landlord will use its reasonable endeavours to provide or procure the provision of the Services PROVIDED THAT the Landlord shall be entitled to withhold or vary the provision or procurement of such of the Services as the Landlord reasonably considers necessary or appropriate in the interests of good estate management and PROVIDED FURTHER THAT the Landlord will not be in breach of this Clause as a result of any failure or interruption of any of the Services:

5.2.1 resulting from circumstances beyond the Landlord's reasonable control, so long as the Landlord uses its reasonable endeavours to remedy the same as soon as reasonably practicable after becoming aware of such circumstances; or

5.2.2 to the extent that the Services (or any of them) cannot reasonably be provided as a result of works of inspection, maintenance and repair or other works being carried out at the Property or the Building or the Centre or the Estate.

6 Insurance

6.1 Landlord's insurance covenants

The Landlord covenants with the Tenant as follows:

6.1.1 To insure the Building (other than tenant's and trade fixtures and fittings) unless the insurance is invalidated in whole or in part by any act or default of the Tenant:

(i) with an insurance office or underwriters of repute;

(ii) against loss or damage by the Insured Risks;

(iii) subject to such excesses as may be imposed by the insurers;

(iv) in the full cost of reinstatement of the Building (in modern form if appropriate) including shoring up, demolition and site clearance, professional fees, VAT and allowance for building cost increases;

6.1.2 To insure against loss of the Principal Rent thereon payable or reasonably estimated by the Landlord to be payable under this lease arising from damage to the Property by the Insured Risks for three years or such longer period as the Landlord may reasonably require having regard to the likely period for reinstating the Property;

6.1.3 The Landlord will use its reasonable endeavours to procure that the insurer waives its rights of subrogation against the Tenant (so long as such provision is available in the London insurance market);

6.1.4 At the request and cost of the Tenant (but not more frequently than once in any twelve month period) to produce summary details of the terms of the insurance under this Clause 6.1;

6.1.5 If the Building is destroyed or damaged by an Insured Risk, then, unless payment of the insurance moneys is refused in whole or part because of the act or default of the Tenant and the Tenant has failed to comply with its obligation in clause 6.2.4(iii) of this lease, and subject to obtaining all necessary planning and other consents to use the insurance proceeds (except those relating to loss of rent and fees) and any uninsured excess paid by the Tenant under Clause 6.2.4(ii) in reinstating the same (other than tenant's and trade fixtures and fittings) as quickly as reasonably practicable in modern form if appropriate but not necessarily identical in layout and (in relation to the Property) substantially as it was before the destruction or damage with the Landlord making up any shortfall in the insurance proceeds out of its own money;

6.2 Tenant's insurance covenants

The Tenant covenants with the Landlord from and including the Insurance Commencement Date and then throughout the Term or until released pursuant to the 1995 Act as follows:

6.2.1 To pay to the Landlord on demand sums equal to:

(i) a fair proportion (reasonably determined by the Landlord's Surveyors) of the amount which the Landlord spends on insurance pursuant to Clause 6.1.1;

(ii) the whole of the amount which the Landlord spends on insurance pursuant to Clause 6.1.2;

(iii) the cost of property owners' liability and third party liability insurance in connection with the Property;

(iv) the cost of any professional valuation of the Property properly required by the Landlord (but not more than once in any two year period);

6.2.2 To give the Landlord immediate written notice on becoming aware of any event or circumstance which might affect or lead to an insurance claim;

6.2.3 Not to do anything at the Property which would or might prejudice or invalidate the insurance of the Building or the Adjoining Property or cause any premium for their insurance to be increased;

6.2.4 To pay to the Landlord on demand:

(i) any increased premium and any Costs incurred by the Landlord as a result of a breach of Clause 6.2.3;

(ii) a fair proportion (reasonably determined by the Landlord's Surveyors) of any uninsured excess to which the insurance policy may be subject;

(iii) the whole of the irrecoverable proportion of the insurance moneys if the Building or any part are destroyed or damaged by an Insured Risk but the insurance moneys are irrecoverable in whole or part due to the act or default of the Tenant;

6.2.5 To comply with the requirements and reasonable recommendations of the insurers;

6.2.6 To notify the Landlord of the full reinstatement cost of any fixtures and fittings installed at the Property at the cost of the Tenant which become Landlord's fixtures and fittings;

6.2.7 Not to effect any insurance of the Property against an Insured Risk but if the Tenant effects or has the benefit of any such insurance the Tenant shall hold any insurance moneys upon trust for the Landlord and pay the same to the Landlord as soon as practicable;

6.3 Suspension of Rent

6.3.1 If the Property (or the means of access thereto) are unfit for occupation or use because of damage by an Insured Risk then (save to the extent that payment of the loss of rent insurance moneys is refused due to the act or default of the Tenant) the Principal Rent (or a fair proportion according to the nature and extent of the damage) shall be suspended until the date on which the Property is again fit for occupation and use and/or accessible.

6.3.2 If clause 6.3.1 applies at any time during the Concessionary Rent Period then the period of suspension of the Principal Rent under clause 6.3.1 shall be adjusted by adding the unexpired portion of the Concessionary Rent Period (calculated pro rata if the rent suspension is applied pro rata) to the date the rent suspension ends;

6.3.3 If the Principal Rent (or a fair proportion according to the nature and extent of the damage) shall be suspended and if either party exercises its right to terminate the Contractual Term pursuant to clause 6.4 or 6.5 of this lease or if this lease terminates in accordance with clause 6.5 of this lease then within 10 working days of such termination the Landlord shall repay to the Tenant any payment of the Principal Rent which relates to a period after the date of termination of the Contractual Term and the Landlord shall also repay to the Tenant any amounts paid by the Tenant pursuant to clause 6.2.1 of this lease and which relate to a period after the date of termination of the Contractual Term;

6.3.4 If the Principal Rent (or a fair proportion according to the nature and extent of the damage) shall be suspended and the Building is reinstated in accordance with clause 6.1.5 of this lease the Landlord shall credit any overpayment of the Principal Rent by the Tenant at the date on which any such rent suspension comes into effect against the next payment of the Principal Rent due from the Tenant following expiry of any such rent suspension.

6.4 Determination Right

6.4.1 If the Property is destroyed or damaged by an Insured Risk such that the Property is unfit for occupation and use and shall not be rendered fit for occupation and use within two years and nine months of the date of such damage then either the Landlord or the Tenant may whilst the Property has not been rendered fit for occupation and use terminate the Contractual Term by giving to the other not less than three (3) months' previous notice in writing.

6.4.2 Termination of this lease pursuant to the provisions of Clause 6.4.1 shall be without prejudice to the liability of either party for any antecedent breach of the covenants and conditions herein contained (save for Clause 6.1.5 which shall be deemed not to have applied).

6.5 Uninsured Risks

6.5.1 For the purposes of this Clause 6.5:

(i) These provisions shall apply from the date on which any Insured Risk becomes an Uninsured Risk but only in relation to the Uninsured Risk;

(ii) References to an Insured Risk becoming an Uninsured Risk shall, without limitation, include the application by insurers of an exclusion, condition or limitation to an Insured Risk to the extent to which such risk thereby is or becomes an Uninsured Risk.

(iii) The Landlord shall notify the Tenant in writing as soon as reasonably practicable after an Insured Risk becomes an Uninsured Risk.

6.5.2 If during the Term the Property (or part thereof or the means of access thereto) shall be damaged or destroyed by an Uninsured Risk so as to make the Property (or part therefore) unfit for occupation or use or inaccessible:

(i) The Principal Rent and the Service Charge or a fair proportion according to the nature and extent of the damage sustained will not be payable until the earlier of the date on which:

(a) The Property shall again be fit for occupation and use excluding fitting out and replacement of contents and made accessible; or

(b) This lease shall be terminated in accordance with Clause 6.5.2(ii) or 6.5.5

(ii) The Landlord may within six (6) months of the date of such damage or destruction serve notice on the Tenant confirming that it will reinstate the Property (a 'Reinstatement Notice') so that the Property shall be fit for occupation and use and made accessible and if the Landlord fails to serve a Reinstatement Notice by the expiry of such prescribed period the lease will automatically end on the date one year after the date of such damage or destruction.

6.5.3 Clause 6.5.2(i) shall not apply if an Insured Risk shall have become an Uninsured Risk owing to the act or default of the Tenant or any person deriving title under the Tenant or their respective agents, employees, licensee, invitees or contractors.

6.5.4 If the Landlord shall have served a Reinstatement Notice the provisions of Clause 6.1.6 shall apply as if the damage had been caused by an Insured Risk

6.5.5 If the Landlord shall have served a Reinstatement Notice and such reinstatement has not been completed by the date two years and nine months of the date of such damage at any time after that date the Landlord or the Tenant may terminate this lease by serving not less than three months' notice on the other stating that it terminates this lease, and if by the end of such notice the Property and/or access to it have been reinstated so that the Property is fit for occupation and use and is accessible the notice shall be void and this lease shall continue in full force and effect.

6.5.6 Service of a Reinstatement Notice shall not oblige the Landlord to replace any Tenant's fitting out works or property belonging to the Tenant or any third party.

7 Provisos

7.1 Forfeiture

If any of the following events occur:

7.1.1 the Tenant fails to pay any of the rents payable under this lease within 21 days of the due date (whether or not formally demanded); or

7.1.2 the Tenant or Guarantor breaches any of its obligations in this lease; or

7.1.3 the Tenant or Guarantor being a company incorporated within the United Kingdom

(i) has an Administration Order made in respect of it; or

(ii) passes a resolution, or the Court makes an Order, for the winding up of the Tenant or the Guarantor, otherwise than a member's voluntary winding up of a solvent company for the purpose of amalgamation or reconstruction previously consented to by the Landlord (consent not to be unreasonably withheld); or

(iii) has a receiver or administrative receiver or receiver and manager appointed over the whole or any part of its assets or undertaking; or

(iv) is struck off the Register of Companies; or

(v) is deemed unable to pay its debts within the meaning of Section 123 of the Insolvency Act 1986; or

7.1.4 proceedings or events analogous to those described in Clause 7.1.3 shall be instituted or shall occur where the Tenant or Guarantor is a company incorporated outside the United Kingdom; or

7.1.5 the Tenant or Guarantor being an individual:

(i) has a bankruptcy order made against him; or

(ii) appears to be unable to pay his debts within the meaning of Section 268 of the Insolvency Act 1986;

then the Landlord may re-enter the Property or any part of the Property in the name of the whole and forfeit this lease and the Term created by this lease shall immediately end, but without prejudice to the rights of either party against the other in respect of any breach of the obligations contained in this lease;

7.2 Notices

7.2.1 All notices under or in connection with this lease shall be given in writing

7.2.2 Any such notice shall be duly and validly served if it is served (in the case of a company) to its registered office or (in the case of an individual) to his last known address;

7.2.3 Any such notice shall be deemed to be given when it is:

(i) personally delivered to the locations listed in Clause 7.2.2; or

(ii) sent by registered post, in which case service shall be deemed to occur on the third Working Day after posting.

7.3 No Implied Easements

The grant of this lease does not confer any rights over the Building or the Centre or the Estate or the Adjoining Property or any other property except those mentioned in Part I of the First Schedule, and Section 62 of the Law of Property Act 1925 is excluded from this lease;

8 Break Clause

8.1 The Tenant may terminate the Contractual Term on the Break Date by giving to the Landlord not less than nine (9) months' previous notice in writing;

8.2 Any notice given by the Tenant shall operate to terminate the Contractual Term only if:

(i) the Principal Rent reserved by this lease has been paid by the Break Date; and

(ii) the Break Payment together with any VAT properly payable thereon at the standard rate for the time being payable has been paid to the Landlord in cleared funds by the date on which such notice is given; and

(iii) the Tenant is not in occupation of any part of the Property; and

(iv) there are no continuing subleases of the Property;

8.3 Upon termination pursuant to clause 8.1 of this lease the Contractual Term shall cease on the Break Date but without prejudice to any claim of either party in respect of any prior breach by the other of the obligations contained in this lease;

8.4 If the Tenant does terminate the Contractual Term on the Break Date the Landlord shall repay to the Tenant within 20 working days of the Break Date any Principal Rent, Service Charge and any payments made by the Tenant pursuant to clause 6.2.1 of this lease which relate to a period which extends beyond the Break Date;

8.5 Time shall be of the essence for the purposes of this Clause.

9 Contracts (Rights of Third Parties) Act 1999

A person who is not a party to this lease has no right under the Contracts (Rights of Third Parties) Act 1999 to enforce any terms of this lease.

Executed by the parties as a **Deed** on the date specified in the Prescribed Clauses.

The First Schedule

Part I - Easements and Other Rights granted

There are granted to the Tenant (in common with others authorised by the Landlord)

1 The right to use the relevant Estate Common Areas and the Centre Common Areas and the Common Parts for access to and from the Property and for all purposes for which they are designed;

2 Free and uninterrupted use of all existing and future Conduits which serve the Property, subject to the Landlord's rights to re-route the same subject to there being no unreasonable interruption of services;

3 The right to enter the Building (excluding the Lettable Units) and the Centre and/or the Estate and/or the Adjoining Property excluding any buildings which are occupied as necessary to perform Clause 4.4 [repair] on reasonable prior written notice to the Landlord, subject to causing as little inconvenience as practicable and complying with conditions reasonably imposed by the Landlord and making good all physical damage caused;

4 The right of support and protection from the remainder of the Building;

5 The right to use such areas of the Building as the Landlord from time to time designates for plant and equipment serving only the Property (subject to approval under Clause 4.11.2);

6 The right to use 26 parking spaces at the Centre in such locations as the Landlord from time to time allocates, the initial allocation shaded in green on Plan 4;

7 The right at all reasonable times and on reasonable notice (which shall not be less than 72 hours' notice except in emergency) to gain entry to the lift motor room shown shaded in grey on Plan 1A in order to repair, maintain, clean, alter or replace any plant and machinery relating to the lift which serves the Property;

8 The right in emergency or as part of any practice fire drill to use the external fire escape staircase leading from the first floor part of the Property to the car park in the Centre;

9 The right to use the balconies at first floor level of the Property;

Part II - Exceptions and Reservations

There are excepted and reserved to the Landlord (and also exercisable by others authorised by the Landlord):

1 The right to carry out any building, rebuilding, alteration or other works to the Building (including, without prejudice to the generality of the foregoing, the Lift Works), the Centre, the Estate and the Adjoining Property (including the erection of scaffolding) notwithstanding any temporary interference with light and air enjoyed by the Property;

2 Free and uninterrupted use of all existing and future Conduits which are in the Property and serve the Building, the Centre, the Estate or the Adjoining Property;

3 Rights of entry on the Property as referred to in Clause 4.18;

4 The right to regulate and control in a reasonable manner the use of the Common Parts, the Centre Common Areas and the Estate Common Areas;

5 The right to alter the layout of the roads forecourts footpaths pavements and car parking areas from time to time at the Centre or on the Estate in such manner as the Landlord may reasonably require PROVIDED THAT such alterations do not materially diminish the Tenant's rights under this lease;

6 The right of support and protection for other parts of the Building;

7 The right in the last six months of the Term to view the Property with prospective tenants upon giving reasonable notice and the right throughout the Term to view the Property with prospective purchasers of the Estate or any part thereof upon giving reasonable notice.

Part III - Encumbrances

The covenants declarations and other matters affecting the Property contained or referred to in the Landlord's freehold reversionary title number BK102078 as at 23 September 2016 at 12:55:40

The Second Schedule

Rent Review

1 In this Schedule:

1.1 **Review Date** means the Review Date;

1.2 **Rack Rental Value** means the annual rent (exclusive of VAT) at which the Property might reasonably be expected to be let in the open market at the Review Date

ASSUMING

1.2.1 the letting is on the same terms as those contained in this lease but subject to the following qualifications:

(i) the term shall commence on the Review Date and be five (5) years;

(ii) the amount of the Principal Rent shall be disregarded; and

(iii) the Concessionary Rent Period shall be disregarded

1.2.2 the Property is available to let as a whole, with vacant possession, by a willing landlord to a willing tenant, without premium;

1.2.3 the Property is ready, fit and available for immediate occupation and use for the Permitted Use;

1.2.4 all the obligations on the part of the Tenant contained in this lease have been fully performed and observed;

1.2.5 no work has been carried out to the Property which has reduced the rental value of the Property;

1.2.6 if the whole or any part of the Property has been destroyed or damaged it has been fully reinstated;

BUT DISREGARDING

1.2.7 any goodwill attached to the Property by reason of any business carried on there;

1.2.8 any effect on rent of the fact that any Tenant and any undertenant is or has been in occupation of the Property;

1.2.9 any effect on rent of any improvements at the Property made with the Landlord's consent by the Tenant or any undertenant, except improvements carried out pursuant to an obligation to the Landlord or at the expense of the Landlord;

PROVIDED THAT the Rack Rental Value shall be that which would be payable after the expiry of any rent free period or concessionary rent period for fitting out (or the receipt of any contribution to fitting out works or other inducement in lieu thereof) which might be given on a letting of the Property, so that no discount reduction or allowance is made to reflect (or compensate the tenant for the absence of) any such rent free or concessionary rent period or contribution or inducement;

1.3 **Revised Rent** means the new Principal Rent following the Review Date pursuant to paragraph 2 of the Second Schedule.

1.4 **Expert** means a surveyor (who shall be a Fellow of the Royal Institution of Chartered Surveyors with at least ten (10) years' experience in the letting and valuation of premises of a similar nature to and situate in the same region as the Property) agreed between the Landlord and the Tenant, or in the absence of agreement nominated on the application of either party by the President for the time being of the Royal Institution of Chartered Surveyors.

2 The Principal Rent shall be reviewed on the Review Date to the higher of:

2.1 the Principal Rent payable immediately before the Review Date (disregarding any suspension or abatement of the Principal Rent); and

2.2 the Rack Rental Value on the Review Date agreed or determined in accordance with this lease.

3 The Rack Rental Value at the Review Date shall be:

3.1 agreed in writing between the Landlord and the Tenant; or

3.2 determined by an Expert (acting as an expert) on the application of either Landlord or Tenant at any time after the Review Date;

4 In the case of determination by an Expert:

4.1 the Expert will be instructed to afford the Landlord and the Tenant the opportunity to make written representations to him and comment upon written representations received by him;

4.2 if an Expert dies, refuses to act or becomes incapable of acting, or if he fails to notify the parties of his determination within 2 months after receiving the last submission delivered to him, either the Landlord or the Tenant may apply to the President to discharge him and appoint another in his place;

4.3 the fees and expenses of the Expert and any VAT thereon shall be paid by the Landlord and the Tenant in such shares as the Expert shall decide (or in equal shares if the Expert does not decide this point); if one party pays all the Expert's fees and expenses, the paying party may recover the other's share from the other party, in the case of the Landlord as arrears of rent.

5 If a Revised Rent is not agreed or determined by the Review Date:

5.1 the Principal Rent payable immediately before the Review Date shall continue to be payable until the Revised Rent is ascertained;

5.2 when the Revised Rent is ascertained:

5.2.1 the Tenant shall pay within 14 days of ascertainment:

(i) any difference between the Principal Rent payable immediately before the Review Date and the Principal Rent which would have been payable had the Revised Rent been ascertained on the Review Date (the **Balancing Payment**); and

(ii) interest on the Balancing Payment at Base Rate from the date or dates when the Balancing Payment or the relevant part or parts would have been payable had the Revised Rent been ascertained on the Review Date;

5.2.2 the Landlord and Tenant shall sign and exchange a memorandum recording the agreed amount of the Revised Rent.

6 Time shall not be of the essence for the purposes of this Schedule.

The Third Schedule

Guarantee

1 The Guarantor covenants with the Landlord as principal debtor:

1.1 that throughout the Term or until the Tenant is released from its covenants pursuant to the 1995 Act:

1.1.1 The Tenant will pay the rents reserved by and perform its obligations contained in this lease;

1.1.2 The Guarantor will indemnify the Landlord on demand against all Costs arising from any default of the Tenant in paying the rents and performing its obligations under this lease;

1.2 the Tenant [(here meaning the Tenant so named in the Prescribed Clauses)] will perform its obligations under any authorised guarantee agreement that it gives with respect to the performance of any of the covenants and conditions in this lease.

2 The liability of the Guarantor shall not be affected by:

2.1 Any time given to the Tenant or any failure by the Landlord to enforce compliance with the Tenant's covenants and obligations;

2.2 The Landlord's refusal to accept rent at a time when it would or might have been entitled to re-enter the Property;

2.3 Any variation of the terms of this lease;

2.4 Any change in the constitution, structure or powers of the Guarantor the Tenant or the Landlord or the administration, liquidation or bankruptcy of the Tenant or Guarantor;

2.5 Any act which is beyond the powers of the Tenant;

2.6 The surrender of part of the Property;

3 Where two or more persons have guaranteed obligations of the Tenant the release of one or more of them shall not release the others.

4 The Guarantor shall not be entitled to participate in any security held by the Landlord in respect of the Tenant's obligations or stand in the Landlord's place in respect of such security.

5 If this lease is disclaimed, and if the Landlord within 6 months of the disclaimer requires in writing the Guarantor will enter into a new lease of the Property at the cost of the Guarantor on the terms of this lease (but as if this lease had continued and so that any outstanding matters relating to rent review or otherwise shall be determined as between the Landlord and the Guarantor) for the residue of the Contractual Term from and with effect from the date of the disclaimer.

6 If this lease is forfeited and if the Landlord within 6 months of the forfeiture requires in writing the Guarantor will (at the option of the Landlord):

6.1 enter into a new lease as in paragraph 5 above with effect from the date of the forfeiture; or

6.2 pay to the Landlord on demand an amount equal to the moneys which would otherwise have been payable under this lease until the earlier of 6 months after the forfeiture and the date on which the Property is fully relet.

6.3 Where the Property is not fully relet during such 6 month period the Landlord will set off against the Guarantor's liability under paragraph 6 of this Schedule any amounts it obtains from the reletting of any part or parts of the Property.

The Fourth Schedule
Service Charge
Part I - Calculation and payment of the Service Charge

1 In this Schedule unless the context otherwise requires:

1.1 **Accounting Date** means 31 December in each year or such other date as the Landlord notifies in writing to the Tenant from time to time;

1.2 **Accounting Year** means the period from but excluding one Accounting Date to and including the next Accounting Date;

1.3 **Estimated Service Charge** means the Landlord's Surveyor's reasonable and proper estimate of the Service Charge for the Accounting Year notified in writing to the Tenant from time to time;

1.4 **Service Cost** means the reasonable and proper costs and expenses paid or incurred by the Landlord in relation to the provision of the Services (including irrecoverable VAT);

1.5 **Tenant's Share** means a fair and reasonable proportion of the Service Cost.

2 The Service Charge shall be the Tenant's Share of the Service Cost in respect of each Accounting Year, and if only part of an Accounting Year falls within the Term the Service Charge shall be the Tenant's Share of the Service Cost in respect of the relevant Accounting Period divided by 365 and multiplied by the number of days of the Accounting Year within the Term.

3 The Landlord shall have the right to adjust the Tenant's Share from time to time to make reasonable allowances for differences in the services provided to or enjoyable by the other occupiers of the Building or the Centre or the Estate.

4 The Tenant shall pay the Estimated Service Charge for each Accounting Year to the Landlord in advance by equal instalments on the Quarter Days, (the first payment for the period from and including the Service Charge Commencement Date to (but excluding) the next Quarter Day after the Service Charge Commencement Date to be made on the Service Charge Commencement Date); and

4.1 If the Landlord's Surveyor does not notify an estimate of the Service Charge for any Accounting Year the Estimated Service Charge for the preceding Accounting Year shall apply; and

4.2 Any adjustment to the Estimated Service Charge after the start of an Accounting Year shall adjust the payments on the following Quarter Days equally.

5 As soon as practicable after the end of each Accounting Year the Landlord shall serve on the Tenant a summary of the Service Cost and a statement of the Service Charge certified by the Landlord's Surveyor which shall be conclusive (save in the case of manifest error).

6 The difference between the Service Charge and the Estimated Service Charge for any Accounting Year (or part) shall be paid by the Tenant to the Landlord within fourteen days of the date of the statement for the Accounting Year, or allowed against the next Estimated Service Charge payment, or after the expiry of the Term refunded to the Tenant.

7 The Tenant shall be entitled by appointment within a reasonable time following service of the Service Charge statement to inspect the accounts maintained by the Landlord and the Landlord's Surveyor relating to the Service Cost and supporting vouchers and receipts at such location as the Landlord reasonably directs.

Part II - Estate Services

In relation to the Estate the provision of the following services or the Costs incurred in relation to:

1 The Common Areas

Repairing, maintaining and (where appropriate) cleaning, lighting and (as necessary) altering renewing, rebuilding and reinstating the Estate Common Areas.

2 Conduits

The repair, maintenance and cleaning and (as necessary) replacement and renewal of all Conduits within the Estate Common Areas.

3 Plant and machinery

Hiring, operating, inspecting, servicing, overhauling, repairing, maintaining, cleaning, lighting and (as necessary) renewing or replacing any plant, machinery, apparatus and equipment from time to time within the Estate Common Areas or used for the provision of services to the Estate and the supply of all fuel and electricity for the same and any necessary maintenance contracts and insurance in respect thereof.

4 Signs

Maintaining and (where appropriate) cleaning and lighting and (as necessary) renewing and replacing the signboards, all directional signs, fire regulation notices, advertisements, bollards, roundabouts and similar apparatus or works.

5 Landscaping

Maintaining, tending and cultivating and (as necessary) re-stocking any garden or grassed areas including replacing plants, shrubs and trees as necessary.

6 Common facilities

Repairing maintaining and (as necessary) rebuilding as the case may be any party walls or fences, party structures, Conduits or other amenities and easements which may belong to or be capable of being used or enjoyed by the Estate in common with any land or buildings adjoining or neighbouring the Estate.

7 Security

Installation, operation, maintenance, repair, replacement and renewal of closed circuit television systems and other security systems.

8 Outgoings

Any existing and future rates, taxes, charges, assessments and outgoings in respect of the Estate Common Areas or any part of them except tax (other than VAT) payable in respect of any dealing with or any receipt of income in respect of the Estate Common Areas.

9 Transport

The provision of a bus service to and from Didcot or such other transport and/or location (if any) deemed necessary by the Landlord.

10 Statutory requirements

The cost of carrying out any further works (after the initial construction in accordance with statutory requirements) to the Estate Common Areas required to comply with any statute.

11 Management and Staff

11.1 The proper and reasonable fees, costs, charges, expenses and disbursements (including irrecoverable VAT) of any person properly employed or retained by the Landlord for or in connection with surveying or accounting functions or the performance of the Estate Services and any other duties in and about the Estate relating to the general management, administration, security, maintenance, protection and cleanliness of the Estate:

11.2 Management costs fees and disbursements in respect of the Estate of 10% of the Service Cost (excluding costs under this clause 11.2).

11.3 Providing staff in connection with the Estate Services and the general management, operation and security of the Estate and all other incidental expenditure including but not limited to:

11.3.1 salaries, National Health Insurance, pension and other payments contributions and benefits;

11.3.2 uniforms, special clothing, tools and other materials for the proper performance of the duties of any such staff;

11.3.3 providing premises and accommodation and other facilities for staff.

12 Enforcement of Regulations

The reasonable and proper costs and expenses incurred by the Landlord in enforcing the rules and regulations from time to time made pursuant to Clause 4.24 provided that the Landlord shall use all reasonable endeavours to recover such costs and expenses from the defaulting party and provided further that there shall be credited against the Service Cost any such costs recovered.

13 Insurances

13.1 Effecting such insurances (if any) as the Landlord may properly think fit in respect of the Estate Common Areas the plant, machinery, apparatus and equipment used in connection with the provision of the Estate Services (including without prejudice those referred to in paragraph 3 above) and any other liability of the Landlord to any person in respect of those items or in respect of the provision of the Estate Services.

13.2 Professional valuations for insurance purposes (but not more than once in any two year period);

13.3 Any uninsured excesses to which the Landlord's insurance may be subject.

14 Generally

Any reasonable and proper costs (not referred to above) which the Landlord may incur in providing such other services and in carrying out such other works as the Landlord may reasonably consider to be reasonably desirable or necessary for the benefit of occupiers of the Estate.

15 Anticipated Expenditure

Establishing and maintaining reserves to meet the future costs (as from time to time estimated by the Landlord's Surveyor) of providing the Estate Services;

16 Borrowing

The costs of borrowing any sums required for the provision of the Estate Services at normal commercial rates available in the open market or if any such sums are loaned by the Landlord or a Group Company of the Landlord interest at Base Rate.

17 VAT

Irrecoverable VAT on any of the foregoing.

Part III - Centre Services

In relation to the Centre, the provision of the following services or the Costs incurred in relation to:

1 Repairs to the Centre (including Conduits)

Repair, renewal, decoration, cleaning and maintenance of the foundations, roof, exterior and structure, the Conduits, plant and equipment (which are not the responsibility of any tenants of the Centre).

2 Centre Common Areas

(a) Repair, renewal, decoration, cleaning, maintenance and lighting of the Centre Common Areas and other parts of the Centre;

(b) Providing and maintaining any plants in the Centre Common Areas;

(c) Providing signs, nameboards and other notices within the Centre including a sign giving the name of the Tenant or other permitted occupier and its location within the Centre.

3 Services

Procuring water, electricity and sewerage services.

4 Fire Fighting and Security

Provision, operation, repair, renewal, cleaning and maintenance of fire alarms, sprinkler systems, fire prevention and fire-fighting equipment and ancillary apparatus and security alarms, apparatus, closed circuit television and systems as the Landlord considers appropriate.

5 Statutory Requirements

All existing and future rates, taxes, charges, assessments and outgoings payable to any competent authority for or in connection with utilities.

6 Management and Staff

6.1 The proper and reasonable fees, costs, charges, expenses and disbursements (including irrecoverable VAT) of any person properly employed or retained by the Landlord for or in connection with surveying or accounting functions or the performance of the Centre Services and any other duties in and about the Centre relating to the general management, administration, security, maintenance, protection and cleanliness of the Centre:

6.2 Management fees and disbursements incurred in respect of the Centre of 10% of the Service Cost (excluding costs under this paragraph 6.2).

6.3 Providing staff in connection with the Centre Services and the general management, operation and security of the Centre and all other incidental expenditure including but not limited to:

(i) salaries, National Health Insurance, pension and other payments contributions and benefits;

(ii) uniforms, special clothing, tools and other materials for the proper performance of the duties of any such staff;

(iii) providing premises and accommodation and other facilities for staff.

7 General

7.1 Establishing and maintaining reserves to meet the future costs (as from time to time estimated by the Landlord's Surveyor) of providing the Centre Services;

7.2 Any reasonable and proper costs (not referred to above) which the Landlord may incur in providing such other services and in carrying out such other works as the Landlord may reasonably consider to be reasonably desirable or necessary for the benefit of occupiers of the Centre.

7.3 The costs of borrowing any sums required for the provision of the Centre Services at normal commercial rates available in the open market or if any such sums are loaned by the Landlord or a Group Company of the Landlord interest at Base Rate.

8 VAT

Irrecoverable VAT on any of the foregoing.

Part IV - Building Services

In relation to the Building, the provision of the following services or the Costs incurred in relation to:

1 Repairs to the Building (including Conduits)

Repair, renewal, decoration, cleaning and maintenance of the foundations, roof, exterior and structure, the Conduits, plant and equipment (which are not the responsibility of any tenants of the Building).

2 Common Parts

(d) Repair, renewal, decoration, cleaning, maintenance and lighting of the Common Parts and other parts of the Building not comprised in the Lettable Units;

(e) Furnishing, carpeting and equipping the Common Parts;

(f) Cleaning the outside of all external windows;

(g) Providing and maintaining any plants, or floral displays in the Common Parts;

(h) Providing signs, nameboards and other notices within the Building including a sign giving the name of the Tenant or other permitted occupier and its location within the Building in the entrance lobby of the Building.

3 Heating etc. services

(a) Providing heating, air conditioning and ventilation other than to the Lettable Units to such standards and between such hours as the Landlord reasonably decides;

(b) Procuring water and sewerage services.

4 Fire Fighting and Security

Provision, operation, repair, renewal, cleaning and maintenance of fire alarms, sprinkler systems, fire prevention and fire fighting equipment and ancillary apparatus and security alarms, apparatus, closed circuit television and systems as the Landlord considers appropriate.

5 Insurance

5.1 Effecting such insurances (if any) as the Landlord may properly think fit in respect of the Common Parts and all Landlord's plant, machinery, apparatus and equipment and any other liability of the Landlord to any person in respect of those items or in respect of the provision of the Building Services;

5.2 Professional valuations for insurance purposes (but not more than once in any two year period);

5.3 Any uninsured excesses to which the Landlord's insurance may be subject.

6 Statutory Requirements

All existing and future rates, taxes, charges, assessments and outgoings payable to any competent authority or for or in connection with utilities except in respect of the Lettable Units.

7 Management and Staff

7.1 The proper and reasonable fees, costs, charges, expenses and disbursements (including irrecoverable VAT) of any person properly employed or retained by the Landlord for or in connection with surveying or accounting functions or the performance of the Building Services and any other duties in and about the Building relating to the general management, administration, security, maintenance, protection and cleanliness of the Building:

7.2 Management fees and disbursements incurred in respect of the Building of 10% of the Service Cost (excluding costs under this Clause 7.2).

7.3 Providing staff in connection with the Building Services and the general management, operation and security of the Building and all other incidental expenditure including but not limited to:

(iv) salaries, National Health Insurance, pension and other payments contributions and benefits;

(v) uniforms, special clothing, tools and other materials for the proper performance of the duties of any such staff;

(vi) providing premises and accommodation and other facilities for staff.

8 General

8.1 Establishing and maintaining reserves to meet the future costs (as from time to time estimated by the Landlord's Surveyor) of providing the Building Services;

8.2 Any reasonable and proper costs (not referred to above) which the Landlord may incur in providing such other services and in carrying out such other works as the Landlord may reasonably consider to be reasonably desirable or necessary for the benefit of occupiers of the Building.

8.3 The costs of borrowing any sums required for the provision of the Services at normal commercial rates available in the open market or if any such sums are loaned by the Landlord or a Group Company of the Landlord interest at Base Rate.

9 VAT

Irrecoverable VAT on any of the foregoing.

LEASE PARTICULARS

Date of Lease	:	17 February 2017
Original Landlord	:	**MEPC MILTON PARK NO. 1 LIMITED** (Company number 5491670) and **MEPC MILTON PARK NO. 2 LIMITED** (Company number 5491806)
Original Tenant	:	**SUMMIT THERAPEUTICS PLC** (Company number 05197494)
Original Guarantor	:	None
Property	:	136A Eastern Avenue, Milton Park
Floor Area		630.0 square metres (6,781 square feet) net internal
Contractual Term	:	Ten (10) years from and including 13 February 2017 to and including 12 February 2027
Initial Principal Rent	:	THIRTY TWO THOUSAND EIGHT HUNDRED AND EIGHTY EIGHT POUNDS (£32,888) per annum
Rent Commencement Date	:	17 February 2017
Review Date	:	17 February 2022
Review Type	:	Market - upwards only
Service Charge Commencement Date	:	17 February 2017
Principal Rent and Service Charge Payment Dates	:	Quarterly: 25 March, 24 June, 29 September and 25 December
Insurance Commencement Date	:	17 February 2017
Permitted Use: (1987 Order)	:	B1 Offices
Break Date	:	17 February 2022
Break Type	:	Tenant - Once only
Parking Spaces	:	26
Security of Tenure: Landlord and Tenant Act 1954	:	Included

EXECUTED AS A DEED by **MEPC MILTON PARK NO. 1 LIMITED** acting by a director and the company secretary or by two directors }

Director

Director/Company Secretary

EXECUTED AS A DEED by **MEPC MILTON PARK NO. 2 LIMITED** acting by a director and the company secretary or by two directors }

Director

Director/Company Secretary

EXECUTED AS A DEED by **SUMMIT THERAPEUTICS PLC** acting by a director and the company secretary or by two directors }

Director

Glyn Edwards - Director

Director/Company Secretary

Melissa Strange
Company Secretary

Exhibit 8.1

SUBSIDIARIES OF THE REGISTRANT

Name of Subsidiary	Jurisdiction of incorporation or organization
Summit Therapeutics Inc.	Delaware, USA
Summit Corporation Limited	England and Wales
Summit (Oxford) Limited	England and Wales
Summit (Wales) Limited	England and Wales
Summit (Cambridge) Limited	England and Wales
Summit Discovery 1 Limited	England and Wales
Summit Corporation Employee Benefit Trust Company Limited	England and Wales
MuOx Limited	England and Wales

Exhibit 12.1

Certification by the Chief Executive Officer
Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002

I, Glyn Edwards, certify that:

1. I have reviewed this annual report on Form 20-F of Summit Therapeutics plc (the "Company");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Company as of, and for, the periods presented in this report;

4. The Company's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Company and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Company, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the Company's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the Company's internal control over financial reporting that occurred during the period covered by the annual report that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting; and

5. The Company's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Company's auditors and the audit committee of the Company's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Company's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal control over financial reporting.

Date: March 30, 2017

By: /S/ Glyn Edwards
Name: Glyn Edwards
Title: Chief Executive Officer

Exhibit 12.2

Certification by the Chief Financial Officer
Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002

I, Erik Ostrowski, certify that:

1. I have reviewed this annual report on Form 20-F of Summit Therapeutics plc (the "Company");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Company as of, and for, the periods presented in this report;

4. The Company's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Company and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Company, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the Company's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the Company's internal control over financial reporting that occurred during the period covered by the annual report that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting; and

5. The Company's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Company's auditors and the audit committee of the Company's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Company's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal control over financial reporting.

Date: March 30, 2017

By: /s/ Erik Ostrowski
Name: Erik Ostrowski
Title: Chief Financial Officer

Exhibit 13.1

Certification by the Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

In connection with the Annual Report on Form 20-F of Summit Therapeutics plc (the "Company") for the year ended January 31, 2017, as filed with the U.S. Securities and Exchange Commission on the date hereof (the "Report"), the undersigned Glyn Edwards, as Chief Executive Officer of the Company, and Erik Ostrowski, as Chief Financial Officer of the Company, each hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 30, 2017

By: /s/ Glyn Edwards
Name: Glyn Edwards
Title: *Chief Executive Officer*

By: /s/ Erik Ostrowski
Name: Erik Ostrowski
Title: *Chief Financial Officer*

Exhibit 15.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the incorporation by reference in the Registration Statement on Form F-3 (No. 333-211301) of Summit Therapeutics plc of our report dated March 29, 2017 relating to the consolidated financial statements, which appears in this Form 20-F.

/s/ PricewaterhouseCoopers LLP

PricewaterhouseCoopers LLP
Reading, UK
March 30, 2017

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