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

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2016

OR

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

For the transition period from to

Commission file number 001-14895

SAREPTA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**93-0797222**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
215 First Street, Suite 415 Cambridge, MA	**02142**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 274-4000

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 ☐

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

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller Reporting Company	☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date.

Common Stock with $0.0001 par value	**54,590,380**
(Class)	**(Outstanding as of November 3, 2016)**

SAREPTA THERAPEUTICS, INC.
FORM 10-Q
INDEX

PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited, in thousands, except shares and per share amounts)

	As of September 30, 2016	As of December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 395,140	$ 80,304
Short-term investments	—	112,187
Accounts receivable	3,986	3,977
Restricted investment	10,695	10,695
Inventory	2,921	—
Other current assets	22,002	17,380
Total current assets	434,744	224,543
Restricted cash and investments	784	783
Property and equipment, net of accumulated depreciation of $28,426 and $24,594 as of September 30, 2016 and December 31, 2015, respectively	35,620	37,344
Intangible assets, net of accumulated amortization of $3,054 and $2,620 as of September 30, 2016 and December 31, 2015, respectively	8,111	6,642
Other non-current assets	8,051	4,470
Total assets	$ 487,310	$ 273,782
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 20,684	$ 20,234
Accrued expenses	29,539	29,053
Current portion of long-term debt	10,107	5,936
Current portion of notes payable	—	2,493
Deferred revenue	3,303	3,303
Other current liabilities	1,300	1,275
Total current liabilities	64,933	62,294
Long-term debt	8,491	14,969
Deferred rent and other	5,262	6,172
Total liabilities	78,686	83,435
Commitments and contingencies (Note 12)		
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 99,000,000 shares authorized; 54,351,725 and 45,629,529 issued and outstanding at September 30, 2016 and December 31, 2015, respectively	5	5
Additional paid-in capital	1,486,487	1,089,508
Accumulated other comprehensive loss	—	(111)
Accumulated deficit	(1,077,868)	(899,055)
Total stockholders' equity	408,624	190,347
Total liabilities and stockholders' equity	$ 487,310	$ 273,782

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2016	2015	2016	2015
Revenues	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	34,349	36,673	117,523	105,018
General and administrative	22,184	15,090	60,812	50,714
Total operating expenses	56,533	51,763	178,335	155,732
Operating loss	(56,533)	(51,763)	(178,335)	(155,732)
Other income (loss):				
Interest (expense) income and other, net	(209)	(176)	(478)	383
Total other (loss) income	(209)	(176)	(478)	383
Net loss	$ (56,742)	$ (51,939)	$ (178,813)	$ (155,349)
Other comprehensive income (loss):				
Unrealized (loss) gain on short-term securities - available-for-sale	(1)	18	111	94
Total other comprehensive (loss) income	(1)	18	111	94
Comprehensive loss	$ (56,743)	$ (51,921)	$ (178,702)	$ (155,255)
Net loss per share — basic and diluted	$ (1.18)	$ (1.25)	$ (3.83)	$ (3.75)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	48,254	41,565	46,709	41,416

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited, in thousands)

	For the Nine Months Ended September 30,	
	2016	**2015**
Cash flows from operating activities:		
Net loss	$ (178,813)	$ (155,349)
Adjustments to reconcile net loss to cash flows from operating activities:		
Depreciation and amortization	3,947	3,883
Amortization of premium on available-for-sale securities, loss from sale of available-for-sale securities and non-cash interest	473	805
Loss on abandonment of patents	45	180
Stock-based compensation	23,093	25,769
Non-cash restructuring expenses	504	—
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(9)	(317)
Net increase in inventory	(2,921)	—
Net (increase) decrease in other assets	(8,203)	9,963
Net decrease in accounts payable, accrued expenses, deferred revenue and other liabilities	(2,703)	(3,127)
Net cash used in operating activities	(164,587)	(118,193)
Cash flows from investing activities:		
Purchase of property and equipment	(2,427)	(2,316)
Purchase of intangible assets	(1,093)	(982)
Purchase of restricted investments	—	(10,695)
Purchase of available-for-sale securities	—	(49,632)
Sale and maturity of available-for-sale securities	112,101	141,854
Net cash provided by investing activities	108,581	78,229
Cash flows from financing activities:		
Proceeds from borrowings, net of debt issuance costs	—	19,601
Repayments of long-term debt and notes payable	(5,076)	(2,573)
Proceeds from sales of common stock	364,951	—
Proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program	10,967	5,204
Net cash provided by financing activities	370,842	22,232
Increase (decrease) in cash and cash equivalents	314,836	(17,732)
Cash and cash equivalents:		
Beginning of period	80,304	73,551
End of period	395,140	55,819
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 1,199	$ 359
Supplemental schedule of non-cash investing activities and financing activities:		
Shares withheld for taxes	$ 1,955	$ —
Accrual for debt issuance costs related to the senior secured term loan	$ 400	$ 400
Intangible assets included in accrued expenses	$ 1,230	$ 105
Accrual for offering costs related to the equity offerings	$ 222	$ 135
Property and equipment included in accrued expenses	$ —	$ 211
Capitalized interest	$ —	$ 99

See accompanying notes to unaudited condensed consolidated financial statements.

1. BUSINESS AND BASIS OF PRESENTATION

Business

Sarepta Therapeutics, Inc. (together with its wholly-owned subsidiaries, "Sarepta" or the "Company") is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. Applying its proprietary, highly-differentiated and innovative platform technologies, the Company is able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy ("DMD") drug candidates. On September 19, 2016, the United States Food and Drug Administration ("FDA") granted accelerated approval for EXONDYS 51, indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is studied in clinical trials under the name of eteplirsen and is marketed in the U.S. under the trademarked name of EXONDYS 51™ (eteplirsen) Injection.

Through September 30, 2016, the Company had not generated any revenue from product sales, and the Company may never generate substantial revenue from product sales. Even if sales of EXONDYS 51 generate substantial revenue, the Company is likely to continue to incur operating losses in the near term.

As of September 30, 2016, the Company had approximately $406.6 million of cash, cash equivalents and restricted cash and investments, consisting of $395.1 million of cash and cash equivalents and $11.5 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of September 30, 2016 is sufficient to fund its current operational plan for the next twelve months, though it may pursue additional cash resources through public or private financings, sell its Priority Review Voucher, seek additional government funding and establish collaborations with or license its technology to other companies.

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP"), reflect the accounts of Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries. All inter-company transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: the development of pharmaceutical products. The information included in this quarterly report on Form 10-Q should be read in conjunction with the Company's consolidated financial statements and the accompanying notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015.

Estimates and Uncertainties

The preparation of the unaudited condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenue, expenses and the disclosure of contingent assets and liabilities. Actual results could differ from those estimates. Significant items subject to such estimates and assumptions include the valuation of stock-based awards, research and development expenses and income taxes.

2. SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

Significant Accounting Policies

For details about the Company's accounting policies, please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* of the Annual Report on Form 10-K for the year ended December 31, 2015. Related to the commercialization of EXONDYS 51, the Company updated its significant accounting policies as follows:

Revenue Recognition

The Company recognizes revenue when all of the following criteria are met:

1) persuasive evidence of an arrangement exists;
2) delivery has occurred or services have been rendered;
3) price to the customer is fixed or determinable; and
4) collectability is reasonably assured.

Revenue from product sales is recognized when title and risk of loss have passed to the customer and is recorded net of applicable reserves for discounts and allowances.

The Company establishes reserves for various government rebate programs and co-payment assistance. Reserves established for these discounts and allowances are classified as either reductions of accounts receivable or a liability. These reserves are based on estimates of the amounts earned or to be claimed on the related sales.

Additionally, the Company also expects to maintain certain customer service contracts with distributors and other customers in the distribution channel that will provide inventory management, data and distribution services, which generally will be reflected as a reduction of revenue. To the extent the Company can demonstrate a separable benefit and fair value for these services, the Company will classify these payments in selling, general and administrative expenses.

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis. The Company capitalizes inventory costs associated with products upon regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Drug products to be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes.

The following table summarizes the components of the Company's inventory for the period indicated:

	As of September 30, 2016
	(in thousands)
Raw materials	$ 2,712
Finished goods	209
Total inventory	$ 2,921

The Company periodically reviews its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. Additionally, though the Company's product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications. As a result, the Company will record a charge to cost of sales to write down any unmarketable inventory to its estimated net realizable value.

Intangible Assets

The Company's intangible assets consist of an in-licensed right and patent costs, which are stated in the Company's consolidated balance sheets net of accumulated amortization and impairments, if applicable.

The in-licensed right relates to the license agreement with the University of Western Australia ("UWA"). As a result of the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement (defined in Note 3), the Company was obligated to pay a $1.0 million sales milestone to UWA and, accordingly, has recorded an in-licensed right. The in-licensed right will be amortized on a straight-line basis over the remaining life of the related patent because the life of the related patent reflects the expected time period that the Company will benefit from the in-licensed right. The amortization of the in-licensed right will be recorded as cost of goods sold in the Company's consolidated statements of operations and comprehensive loss.

The following table summarizes the components of intangible assets for the period indicated:

	As of September 30, 2016
	(in thousands)
Patent costs	$ 10,165
In-licensed right	1,000
Intangible assets, gross	$ 11,165
Less: accumulated amortization	(3,054)
Intangible assets, net	$ 8,111

There have not been any other material changes to the Company's accounting policies as of September 30, 2016.

Recent Accounting Pronouncements

In August 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-15, "*Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments*". The amendments in this update clarify how certain cash receipts and cash payments are presented and classified in the statement of cash flows. ASU No. 2016-15 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. As of September 30, 2016, the Company has not elected to early adopt this guidance and does not expect the adoption of this guidance to have any impact on its consolidated financial statements.

In March 2016, the FASB issued ASU No. 2016-09, "*Improvements to Employee Share-Based Payment Accounting*". The amendments in this update simplify several aspects of the accounting for share-based payment transactions, including income tax consequences, classification of awards as either equity or liabilities and classification on the statement of cash flows. ASU No. 2016-09 will be effective for fiscal years beginning after December 15, 2016, with early adoption permitted. As of September 30, 2016, the Company has not elected to early adopt this guidance but determined that the adoption of this standard will not have any impact on the Company's financial statements.

In February 2016, the FASB issued ASU No. 2016-02, "*Leases (Topic 842)*", which supersedes Topic 840, "*Leases*". Under the new guidance, a lessee should recognize assets and liabilities that arise from its leases and disclose qualitative and quantitative information about its leasing arrangements. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. As of September 30, 2016, the Company has not elected to early adopt this guidance or determined the effect that the adoption of this guidance will have on its consolidated financial statements.

In July 2015, the FASB issued ASU No. 2015-11, "*Inventory (Topic 330): Simplifying the Measurement of Inventory"*. The new standard applies only to inventory for which cost is determined by methods other than last-in, first-out and the retail inventory method, which includes inventory that is measured using first-in, first-out or average cost. Inventory within the scope of this standard is required to be measured at the lower of cost and net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The new standard will be effective for fiscal years beginning after December 15, 2016. As of September 30, 2016, the Company has not elected to early adopt this guidance but determined that the adoption of this standard will not have any impact on the Company's financial statements.

In August 2014, the FASB issued ASU No. 2014-15, "*Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*". This update requires an entity's management to evaluate whether there are conditions or events, considered in the aggregate, that raise substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued and to provide related disclosures. ASU No. 2014-15 will be effective for the fiscal years beginning after December 15, 2016, with early adoption permitted. As of September 30, 2016, the Company has not elected to early adopt this guidance, and based on the Company's financial condition as of the date these financial statements were issued or available for issuance, the Company does not expect the adoption of this guidance to have any impact on the current period financial statements.

In May 2014, the FASB issued ASU No. 2014-09, "*Revenue from Contracts with Customers (Topic 606)*". This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, "*Revenue Recognition*". Under the new guidance, a company is required to recognize revenue when it transfers goods or renders services to customers at an amount that it expects to be entitled to in exchange for these goods or services. The new standard allows for either a full retrospective with or without practical expedients or a retrospective with a cumulative catch upon adoption transition method. This guidance is effective for the fiscal years beginning after December 15, 2016, with early adoption not permitted. In August 2015, the FASB issued ASU No. 2015-14, "*Deferral of the Effective Date*", which states that the mandatory effective date of this new revenue standard will be delayed by one year, with early adoption only permitted in fiscal year 2017. During the second quarter of 2016, the FASB issued three amendments to the new revenue standard to address some application questions: ASU No. 2016-10, "*Identifying Performance Obligations and Licensing*", ASU No. 2016-11, "*Rescission of SEC Guidance Because of Accounting Standards Updates 2014-09*", and ASU No. 2016-12, "*Narrow-Scope Improvements and Practical Expedients*". These three amendments will be effective upon adoption of Topic 606. As of September 30, 2016, the Company has not yet determined which adoption method it will utilize or the effect that the adoption of this guidance will have on its consolidated financial statements.

3. SIGNIFICANT AGREEMENTS

University of Western Australia

In April 2013, the Company and UWA entered into an agreement under which an existing exclusive license agreement between the Company and UWA was amended and restated (the "Amended and Restated UWA License Agreement"). The Amended and Restated UWA License Agreement grants the Company specific rights to the treatment of DMD by inducing the skipping of certain exons. EXONDYS 51 falls under the scope of the license agreement. Under the Amended and Restated UWA License Agreement, the

Company may be required to make payments of up to $6.0 million in aggregate to UWA based on the successful achievement of certain development and sales milestones relating to EXONDYS 51 and up to five additional product candidates. The Company may also be obligated to make payments to UWA of up to $20.0 million upon the achievement of certain sales milestones. Additionally, the Company may also be required to pay a low-single-digit percentage royalty on net sales of products covered by issued patents licensed from UWA during the term of the Amended and Restated UWA License Agreement. However, the Company has the option to purchase future royalties up-front. Under this option, prior to the First Amendment (defined below), the Company could be required to make a one-time royalty payment of $30.0 million to UWA.

In June 2016, the Company and UWA entered into the first amendment to the Amended and Restated UWA License Agreement (the "First Amendment"). Under the First Amendment, the Company was obligated to make an up-front payment of $7.0 million to UWA upon execution of the amendment. Under the terms of the First Amendment, UWA has waived certain rights and amended the timing of certain payments under the Amended and Restated UWA License Agreement, including lowering the up-front payment that is due by the Company upon exercise of the option to purchase future royalties up-front. Upon exercise of the option to purchase future royalties up-front, the Company would still be obligated to make up to $20.0 million in payments to UWA upon achievement of certain sales milestones.

For the three and nine months ended September 30, 2016, the Company recorded $0.3 million and $7.3 million, respectively, relating to the development milestone and up-front payments to UWA as research and development expense in the unaudited condensed consolidated statement of operations and comprehensive loss.

Additionally, corresponding to the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement, the Company recorded a $1.0 million milestone as an in-license right in the unaudited condensed consolidated balance sheets as of September 30, 2016.

4. FAIR VALUE MEASUREMENTS

The Company has certain financial assets that are recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements.

- Level 1 — quoted prices for identical instruments in active markets;
- Level 2 — quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active, and model-derived valuations in which all significant inputs and significant value drivers are observable in active markets; and
- Level 3 — valuations derived from valuation techniques in which one or more significant value drivers are unobservable.

The tables below present information about the Company's financial assets that are measured and carried at fair value and indicate the level within the fair value hierarchy of valuation techniques it utilizes to determine such fair value:

	Fair Value Measurement as of September 30, 2016			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 36,612	$ 36,612	$ —	$ —
Certificates of deposit	11,343	11,343	—	—
Total assets	$ 47,955	$ 47,955	$ —	$ —

	Fair Value Measurement as of December 31, 2015			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 32,850	$ 32,850	$ —	$ —
Commercial paper	48,899	—	48,899	—
Government and government agency bonds	50,918	—	50,918	—
Corporate bonds	17,370	—	17,370	—
Certificates of deposit	11,343	11,343	—	—
Total assets	$ 161,380	$ 44,193	$ 117,187	$ —

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds and certificates of deposit. Money market funds are publicly traded mutual funds and are presented as cash equivalents in the unaudited condensed consolidated balance sheets as of September 30, 2016.

The Company's assets with fair value categorized as Level 2 within the fair value hierarchy consist of commercial paper, government and government agency bonds and corporate bonds. These assets have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, through income-based approaches utilizing observable market data.

The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents, accounts receivable and accounts payable approximate fair value because of the immediate or short-term maturity of these financial instruments. The carrying amounts for long-term debt approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

5. CASH, CASH EQUIVALENTS AND SHORT-TERM INVESTMENTS

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. As of September 30, 2016, the Company did not hold any available-for-sale securities. The weighted average maturity of the Company's available-for-sale securities as of December 31, 2015 was approximately four months.

The following tables summarize the Company's cash, cash equivalents and short-term investments for each of the periods indicated; as of September 30, 2016, there were no short-term investments.

	As of December 31, 2015			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 75,304	$ —	$ —	$ 75,304
Commercial paper	48,936	—	(37)	48,899
Government and government agency bonds	50,966	—	(48)	50,918
Corporate bonds	17,396	—	(26)	17,370
Total assets	$ 192,602	$ —	$ (111)	$ 192,491
As reported:				
Cash and cash equivalents	$ 80,304	$ —	$ —	$ 80,304
Short-term investments	112,298	—	(111)	112,187
Total assets	$ 192,602	$ —	$ (111)	$ 192,491

6. OTHER CURRENT ASSETS AND OTHER NON-CURRENT ASSETS

The following table summarizes the Company's other current assets for each of the periods indicated:

	As of September 30, 2016	As of December 31, 2015
	(in thousands)	
Manufacturing-related deposits	$ 16,783	$ 13,070
Prepaid expenses	3,663	3,109
Other	1,556	1,201
Total other current assets	$ 22,002	$ 17,380

The following table summarizes the Company's other non-current assets for each of the periods indicated:

	As of September 30, 2016	As of December 31, 2015
	(in thousands)	
Manufacturing-related deposits	$ 4,084	—
Prepaid clinical expenses	3,725	4,228
Other	242	242
Total other non-current assets	$ 8,051	$ 4,470

7. ACCRUED EXPENSES

The following table summarizes the Company's accrued expenses for each of the periods indicated:

	As of September 30, 2016	As of December 31, 2015
	(in thousands)	
Accrued clinical and preclinical costs	$ 9,094	$ 9,587
Accrued employee compensation costs	7,649	8,189
Accrued contract manufacturing costs	7,605	4,830
Accrued professional fees	2,280	4,258
Accrued research costs	1,746	629
Other	1,165	1,560
Total accrued expenses	$ 29,539	$ 29,053

8. EQUITY FINANCING

In September 2016, the Company sold approximately 5.8 million shares of common stock through an underwritten public offering at a price of $59.75 per share. As of the date of the issuance of this report, the Company received aggregate net proceeds of approximately $327.4 million from the offering net of commission and offering expenses of approximately $17.6 million.

In June 2016, the Company sold approximately 2.1 million shares of common stock through an underwritten public offering at a price of $17.84 per share. The implied underwriting discount and commission was $1.60 per share. The Company received aggregate net proceeds of approximately $37.3 million from the offering net of offering expense of approximately $0.2 million.

9. RESTRUCTURING

In March 2016, the Company announced a long-term plan ("Corvallis plan") to consolidate all of the Company's operations to Massachusetts and reduce its workforce by approximately 19% as part of a strategic plan to increase operational efficiency. During the remainder of the year, the Company plans to close its facility in Corvallis, Oregon, which primarily focused on early-stage research and research manufacturing. As part of the consolidation, research activities and some employees will transition to the Company's facilities in Andover and Cambridge, Massachusetts. The consolidation efforts are planned to occur in four waves - May, October, November and December of 2016, with an estimated completion date of December 30, 2016. The restructuring costs of the Corvallis plan consist of costs associated with its workforce reduction and facility consolidation. The workforce reduction costs primarily relate to employee severance and benefits. Facility consolidation costs are primarily associated with non-cancellable lease obligations as well as accelerated depreciation for certain assets whose expected useful lives are shortened due to the consolidation. The Company has not determined the financial impact related to the non-cancellable lease obligation for the Corvallis facility but is currently obligated to make $4.3 million of lease payments after the estimated completion date of the consolidation plan. The Company estimates restructuring expenses of $1.8 million related to accelerated depreciation and workforce reduction costs, the latter of which will be accrued as earned over the service period for each employee.

In August 2016, the Company implemented a restructuring plan in Cambridge, Massachusetts ("Cambridge plan") and reduced its workforce by approximately 6%. The restructuring costs associated with the Cambridge plan consist of costs associated with workforce reduction totaling $0.7 million. The Cambridge plan was completed as of October 31, 2016.

For the three and nine months ended September 30, 2016, the Company recognized $1.3 million and $2.4 million of restructuring expenses, respectively, $1.0 million and $2.1 million, respectively, of which related to workforce reduction.

The following table summarizes the restructuring costs by function for the periods indicated:

	For the Three Months Ended September 30, 2016			For the Nine Months Ended September 30, 2016		
			(in thousands)			
	Cash	Non-cash (1)	Total	Cash	Non-cash (2)	Total
Research and development	$ 628	$ 143	$ 771	$ 1,448	$ 336	$1,784
General and administration	367	126	493	471	168	639
Total restructuring expenses	$ 995	$ 269	$ 1,264	$ 1,919	$ 504	$2,423

(1) The non-cash restructuring expense relates to accelerated depreciation for certain assets.
(2) The non-cash restructuring expense relates to acceleration of stock option vesting and accelerated depreciation for certain assets.

The following table summarizes the restructuring reserve for the periods indicated:

	For the Three Months Ended September 30, 2016	For the Nine Months Ended September 30, 2016
	(in thousands)	
Restructuring reserve beginning balance	$ 371	$ —
Restructuring expenses incurred during the period	990	1,919
Adjustments to prior period estimates, net	5	—
Amounts paid during the period	(458)	(1,011)
Restructuring reserve ending balance	$ 908	$ 908

10. STOCK-BASED COMPENSATION

The following table summarizes the Company's stock awards granted for each of the periods indicated:

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2016		2015		2016		2015	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	1,050	$ 37.38	702,067	$ 24.05	1,214,426 (1)	$ 11.96	2,676,778	$ 14.67
Restricted stock awards	91,778 (2)	$ 48.94	65,000	$ 33.81	117,553 (3)	$ 41.22	181,783	$ 20.80

(1) Included in 2016 stock option grants are 287,500 options with performance conditions. As a result of the approval of EXONDYS 51, 25% of these performance grants vested immediately and another 25% were triggered to be eligible for vesting subject to the remaining service conditions of the awards. As of September 30, 2016, the performance conditions of the remaining 50% were not probable of being achieved. The remaining stock options granted during the periods presented in the table have only service-based criteria and vest over four years.
(2) The Company granted certain employees 91,778 restricted stock awards ("RSA") with certain sales targets. If and when deemed probable that such performance milestones may be achieved within the required time frame, the Company may recognize up to $4.5 million of stock-based compensation related to these grants.
(3) Included in the 2016 RSA grants are 18,755 shares granted to certain employees in lieu of a portion of their 2015 annual bonus payments. These RSA grants were fully vested as of September 30, 2016. The remaining RSAs will be fully vested by June 2017.

Stock-based Compensation Expense

For the three months ended September 30, 2016 and 2015, total stock-based compensation expense was $9.6 million and $5.7 million, respectively. For the nine months ended September 30, 2016 and 2015, total stock-based compensation expense was $23.1 million and $25.8 million, respectively. As a result of the FDA approval of EXONDYS 51, certain performance criteria for options

with performance conditions were met during the quarter. The Company recognized approximately $3.7 million in stock-based compensation expense related to these options. Included in the amount for the nine months ended September 30, 2015 is $8.6 million of stock-based compensation expense incurred in connection with the resignation of the Company's former Chief Executive Officer. The following table summarizes stock-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2016	2015	2016	2015
	(in thousands)			
Research and development	$ 2,674	$ 2,631	$ 7,527	$ 7,639
General and administrative	6,899	3,052	15,566	18,130
Total stock-based compensation expense	$ 9,573	$ 5,683	$ 23,093	$ 25,769

The following table summarizes stock-based compensation expense by grant type included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2016	2015	2016	2015
	(in thousands)			
Stock options	$ 8,778	$ 4,801	$ 20,248	$ 23,451
Restricted stock awards	232	136	689	310
Stock appreciation rights	115	115	345	377
Employee stock purchase plan	448	631	1,811	1,631
Total stock-based compensation expense	$ 9,573	$ 5,683	$ 23,093	$ 25,769

11. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. Given that the Company generated a net loss for each of the periods presented, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and, therefore, would be excluded from the diluted net loss per share calculation.

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2016	2015	2016	2015
	(in thousands, except per share amounts)			
Net loss	$ (56,742)	$ (51,939)	$ (178,813)	$ (155,349)
Weighted-average number of shares of common stock and common stock equivalents outstanding:				
Weighted-average number of shares of common stock outstanding for computing basic loss per share	48,254	41,565	46,709	41,416
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	—	—	—
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	48,254	41,565	46,709	41,416
Net loss per share — basic and diluted	$ (1.18)	$ (1.25)	$ (3.83)	$ (3.75)

* For the three and nine months ended September 30, 2016 and 2015, stock options, RSAs and stock appreciation rights to purchase approximately 6.3 million and 7.3 million shares of common stock, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

12. COMMITMENTS AND CONTINGENCIES

Litigation

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. The Company records a liability in its consolidated financial statements for loss contingencies related to litigation when a loss is known or considered probable and the amount can be reasonably estimated. If the reasonable estimate of a known or probable loss is a range, and no amount within the range is a better estimate than any other, the minimum amount of the range is accrued. If a loss is reasonably possible but not known or probable, and can be reasonably estimated, the estimated loss or range of loss is disclosed.

On January 27, 2014 and January 29, 2014, purported class action complaints were filed in the U.S. District Court for the District of Massachusetts against the Company and certain of its current or former officers. The complaints were consolidated into a single action (Corban v. Sarepta, et al., No. 14-cv-10201) ("Corban") by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. The plaintiffs' consolidated amended complaint, filed on July 21, 2014, sought to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleged that Sarepta and certain of its current or former officers violated the federal securities laws in connection with disclosures related to eteplirsen and sought damages in an unspecified amount. On March 31, 2015, the Court granted Sarepta's motion to dismiss the plaintiffs' amended complaint. On August 12, 2015, the Court denied the plaintiffs' April 30, 2015 motion for leave seeking to file a further amended complaint, and on September 22, 2015, the Court dismissed the case. The plaintiffs filed a Notice of Appeal in the Court of Appeals for the First Circuit on September 29, 2015. On January 27, 2016, the plaintiffs filed a motion to vacate the District Court's order denying leave to amend and dismissing the case, during the pendency of which the plaintiffs' appeal was stayed. On April 21, 2016, the Court denied that motion. On May 19, 2016, the plaintiffs filed a motion to alter or amend the judgment. The Court denied that motion on May 20, 2016. A briefing schedule for the plaintiffs' appeal has been set by the First Circuit. An estimate of the possible loss or range of loss cannot be made at this time.

Another purported class action complaint was filed on December 3, 2014 in the U.S. District Court for the District of Massachusetts (*Kader v. Sarepta et.al* 1:14-cv-14318) ("Kader"), asserting that the Company and certain of its current or former officers violated Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5. The plaintiffs' amended complaint, filed on March 20, 2015, alleged that the defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of an NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. The plaintiffs sought compensatory damages and fees. On April 5, 2016, the Court granted Sarepta's motion to dismiss the amended complaint. On April 8, 2016, the plaintiffs filed a motion for leave to further amend the complaint, which Sarepta opposed on April 22, 2016. That motion remains pending. An estimate of the possible loss or range of loss cannot be made at this time.

On February 5, 2015, a derivative suit was filed in the 215th Judicial District of Harris County, Texas against the Company's Board of Directors (*David Smith, derivatively on behalf of Sarepta Therapeutics, Inc., v. Christopher Garabedian et. al, Case No. 2015-06645*). The claims allege that Sarepta's directors caused Sarepta to disseminate materially false and/or misleading statements in connection with disclosures concerning the Company's submission of the NDA for eteplirsen. Plaintiff seeks unspecified compensatory damages, actions to reform and improve corporate governance and internal procedures, disgorgement of profits, benefits and other compensation obtained by the directors, and attorneys' fees. The parties have agreed to stay the case pending resolution of the Corban and Kader cases. An estimate of the possible loss or range of loss cannot be made at this time.

On March 16, 2016 in the U.S. District Court for the District of Massachusetts against the Company's Board of Directors (*Dawn Cherry, on behalf of nominal defendant Sarepta Therapeutics, Inc., vs. Behrens et. al., 1:16-cv-10531*). The claims allege that the defendants authorized the Company to make materially false and misleading statements about the Company's business prospects in connection with its development of eteplirsen from July 10, 2013 to the present. Plaintiffs seek unspecified damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. The parties have agreed to stay the case pending resolution of the Corban and Kader cases. An estimate of the possible loss or range of loss cannot be made at this time.

Additionally, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (*Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et. al vs. Goolsbee et. al., No. 10157*). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's former CEO, Christopher Garabedian, was also excessive and such fees were the basis for Mr. Garabedian's not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, among others, includes disgorgement and

rescindment of allegedly excessive or unfair payments and equity grants to Mr. Garabedian and the directors, unspecified damages plus interest, a declaration that the Company's Amended and Restated 2011 Equity Plan at the 2013 annual meeting was ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. The parties have agreed to a Memorandum of Understanding concerning the settlement terms and do not believe that disposition of the McDonald suit will have a material financial impact on the Company. The parties are now engaged in the confirmatory discovery process that, when complete, will allow plaintiffs' counsel to represent to the court that the terms of the settlement are fair. Defendants have provided documents to plaintiffs, who are now in the process of reviewing the materials.

13. SUBSEQUENT EVENT

On October 3, 2016, the Company entered into an exclusive Collaboration and License Agreement (the "Collaboration Agreement") with Summit (Oxford) Ltd ("Summit") which grants the Company the exclusive right to commercialize products in Summit's utrophin modulator pipeline in the E.U., Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States (the "Licensed Territory").

Under the terms of the Collaboration Agreement, the Company made an up-front payment of $40.0 million to Summit, with additional payments of up to $192.0 million based on achievement of certain development and regulatory milestones for ezutromid. For Summit's second generation and future generation small molecule utrophin modulators, the Company may be required to make up to $290.0 million in development and regulatory milestone payments. Additionally, on a product-by-product basis, the Company may be required to make up to $330.0 million in sales milestone payments.

The Collaboration Agreement also grants the Company an option to expand the Licensed Territory. If the Company exercises this option, it will be liable for a one-time $10.0 million option fee as well as up to $7.0 million in regulatory milestone payments. For each licensed product, the Company may be liable for up to $82.5 million in sales milestone payments.

Additionally, the Company may be required to make tiered royalty payments ranging from a low to high teens percentage of net sales on a product-by-product basis in the Licensed Territory.

Under the Collaboration Agreement, Summit will be solely responsible for all research and development costs for the licensed products until December 31, 2017. Thereafter, Summit will be responsible for 55.0% of the budgeted research and development costs related to the licensed products in the Licensed Territory, and the Company 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. Summit is also obligated to spend a specified minimum amount on the research and development of certain licensed products prior to the end of 2019.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2015 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion contains certain forward-looking statements, which are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "estimate," "could," "continue," "ongoing," "predict," "potential," "likely," "seek" and other similar expressions, as well as variations or negatives of these words. These statements contain projections of future results of operations or financial condition, or state other "forward-looking" information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

- *the timing, investment and associated activities, including negotiating and entering into any additional commercial and supply contracts, scaling up manufacturing and hiring any additional personnel in connection with commercialization of EXONDYS 51 in the U.S.;*
- *Our expectations regarding the market size for EXONDYS 51 and our ability to manufacture sufficient amounts of EXONDYS 51 to meet actual commercial demand;*
- *our ability to verify the clinical benefit of EXONDYS 51 through our confirmatory trial(s) to obtain full approval from the United States Food and Drug Administration's ("FDA") and/or other regulatory authorities;*
- *third-party payor reimbursement and establishing and maintaining the marketing and distribution support for EXONDYS 51;*
- *our expectations regarding the timing of research, development, pre-clinical and clinical trial results, data and analyses relating to the safety profile and potential clinical benefits of EXONDYS 51 and our product candidates, our phosphorodiamidate morpholino oligomer ("PMO") chemistries, our other PMO-based chemistries and our other RNA-targeted technologies;*
- *Our ability to submit a Marketing Authorization Application ("MAA") for EXONDYS 51in the E.U. by end of year and obtain an approval from the European Medicines Agency ("EMA");*
- *our expectations regarding the FDA's interpretation of our data and information on our product candidates, PMO and PMO-based chemistries and RNA-targeted technologies and the impact on our business of the FDA's interpretations on our FDA submissions (including our investigational new drug ("IND") and new drug application ("NDA")), filing decisions by the FDA, advisory committee recommendations, and FDA product approval decisions and related timelines;*
- *our ability to respond to FDA requests during the regulatory process for each of our product candidates;*
- *our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and business plans and statements about our future capital needs;*
- *our ability to raise additional funds to support our business plans, including business development, and the impact of our credit and security agreement with MidCap Financial on our financial condition and future operations;*
- *our expectations regarding our ability to become a leading developer and marketer of PMO-based and RNA-targeted therapeutics and commercial viability of EXONDYS 51, as well as our product candidates, chemistries and technologies;*
- *the potential safety, efficacy, potency and utility of our product candidates, chemistries and technologies in the treatment of Duchenne muscular dystrophy ("DMD") and other diseases;*
- *our expectations regarding the timing, completion and receipt of results from our ongoing development programs for our pipeline of product candidates including their potential consistency with prior results;*
- *our ability to effectively manage and execute post-marketing approval requirements for EXONDYS 51 and the clinical trial process for our product candidate, including our ability to successfully conduct our placebo-controlled study, ESSENCE using exon 45- and exon 53-skipping product candidates;*

- *our expectations regarding our ability to engage a number of manufacturers with sufficient capability and capacity to meet our manufacturing needs, including with respect to the manufacture of subunits, drug substance APIs and drug product, within the time frames and quantities needed to provide our product candidates to patients in larger scale clinical trials or in commercial quantities, and meet regulatory and Company quality control requirements;*

- *the impact of regulations as well as regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations;*

- *our expectations regarding the potential markets for our product candidates;*

- *our expectations regarding manufacturing and scale-up techniques to support the commercialization of EXONDYS 51;*

- *our expectations regarding our manufacturing and scale-up techniques and our ability to synthesize and purify our product candidates to adequately support clinical development and their potential commercialization;*

- *the potential acceptance of EXONDYS 51 and our product candidates, when introduced, in the marketplace;*

- *the possible impact of any competing products on the commercial success of EXONDYS 51 and our product candidates and our ability to compete against such products;*

- *the impact of potential difficulties in product development, manufacturing, or the commercialization of EXONDYS 51 and our product candidates, including difficulties in establishing and maintaining an appropriate commercial infrastructure necessary for the successful commercialization of EXONDYS 51;*

- *our expectations regarding the partnering opportunities and other strategic transactions that the Company has entered into or considers entering into in the future;*

- *the extent of protection that our patents provide and our pending patent applications may provide, if patents issue from such applications, to our technologies and programs, and our ability to maintain patent protection for our technologies and programs;*

- *our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;*

- *our ability to invalidate some or all of the claims of patents issued to competitors and pending patent applications if issued to competitors, and the potential impact of those claims on the potential commercialization of our product candidates;*

- *our ability to successfully challenge the patent positions of our competitors and successfully defend our patent positions in the actions that the United States Patent and Trademark Offer (the "USPTO") or any appeals court may take or has taken with respect to our patent claims or those of third parties, including any appeals in connection with the recent interference decisions regarding our patents and patent applications and those held by BioMarin Pharmaceuticals, Inc., ("BioMarin") relating to EXONDYS 51 and SRP-4053 and our expectations regarding the impact of any appeals in connection with these interferences on our business plans, including our commercialization for EXONDYS 51 and, if approved by regulatory authorities, SRP-4053;*

- *the impact of any consequences of the interference decisions including the final refusal of BioMarin claims in the exon 51 and exon 53 composition of matter interferences and the narrow claim BioMarin was allowed to pursue as a result of the exon 53 inference decision;*

- *the potential impact if the USPTO, other agencies or courts make a decision against us that could negatively impact the EXONDYS 51 commercialization such as a decision in the pending appeal of Interference No. 106,013 which could result in an infringement claim against us if the patents subject to the appeal are ultimately granted;*

- *our ability to operate our business without infringing the intellectual property rights of others;*

- *our ability to enter into contracts, including collaborations or licensing agreements, with respect to our technology and product candidates, with third parties, including government entities;*

- *our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;*

- *the timing and outcomes of ongoing interference proceedings and related appeals;*
- *the impact of any litigation on us, including actions brought by stockholders;*
- *our ability to attract and retain key employees needed to execute our business plans and strategies and our expectations regarding our ability to manage the impact of any loss of key employees;*
- *our ability to comply with applicable environmental laws and regulations;*
- *our expectations relating to potential funding from government and other sources for the development of some of our product candidates;*
- *the impact of the potential achievement of performance conditions and milestones relating to our stock awards; and*
- *our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements.*

We undertake no obligation to update any of the forward-looking statements contained in this Quarterly Report on Form 10-Q after the date of this report, except as required by law or the rules and regulations of the U.S. Securities and Exchange Commission ("SEC"). We caution readers not to place undue reliance on forward-looking statements. Our actual results could differ materially from those discussed in this Quarterly Report on Form 10-Q. The forward-looking statements contained in this Quarterly Report on Form 10-Q, and other written and oral forward-looking statements made by us from time to time, are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements, including the risks, uncertainties and assumptions identified under the heading "Risk Factors" in this Quarterly Report on Form 10-Q.

Overview

We are a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying pipeline of DMD drug candidates. On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51, indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is studied in clinical trials under the name of eteplirsen and is marketed in the U.S. under the trademarked name of EXONDYS 51™ (eteplirsen) Injection.

Our RNA-targeted technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. EXONDYS 51 is the first approved disease-modifying therapy for DMD in the U.S. and is also our first product candidate to receive marketing approval from the FDA. As of the date of this report, EXONDYS 51 has not been approved for sale or marketing by any regulatory agency or authority outside of the U.S.

We are in the process of conducting, starting, or planning several studies in the U.S. and in Europe for EXONDYS 51 and other product candidates designed to skip exons 45 and 53. These are comprised of:

(i) studies we are currently conducting to further evaluate EXONDYS 51, including an open label extension of our Phase IIb study for which patients can transition to commercial drug after certain criteria are met, the PROMOVI study (an open label study on ambulatory patients with a concurrent untreated control arm), a study on participants with advanced stage DMD and a study on participants with early stage DMD each of which will allow for patients to transition to commercial drug after meeting certain criteria;

(ii) EXONDYS 51 studies we are planning to initiate to comply with U.S. and/ or E.U. regulatory requirements for NDAs and marketing authorization applications, respectively (e.g. an IV study on participants between the ages of six months and four years in connection with our Pediatric Investigation Plan in the E.U., and two additional phase I studies);

(iii) studies we are planning to fulfill our post-marketing FDA requirements for EXONDYS 51 including a 2-year randomized double-blind, controlled trial in patients who have a confirmed mutation of the DMD gene amenable to exon 51 skipping and a 2-year controlled study in patients with confirmed mutations amenable to exon 45 or exon 53 that includes two well separated dose levels for each of the exon 45 skipping product candidate and the exon 53 skipping product candidate;

(iv) a dose-ranging study that we completed for our product candidate designed to skip exon 45 that has transitioned into an open-label study;

(v) a two-part randomized, double-blind, placebo-controlled, dose titration safety, tolerability and pharmacokinetics study for a product candidate designed to skip exon 53 for which we have completed Part I and have now transitioned into Part II, an open label efficacy and safety study; and

(vi) ESSENCE, a placebo-controlled study with product candidates designed to skip exons 45 and 53 which has begun enrolling patients in the U.S. and for which we plan to have sites in the E.U. and Canada.

In addition to our DMD program, we also have leveraged the capabilities of our RNA-targeted technology platforms to develop therapeutic candidates for the treatment of infectious diseases such as influenza, Marburg and Ebola under prior contracts with the U.S. Department of Defense ("DoD"); however, further development of these product candidates would be conditioned, in part, on obtaining additional funding, collaborations or emergency use. Our discovery and research programs include collaborations with various third parties and focus on developing therapeutics in rare, genetic, anti-bacterial, neuromuscular and central nervous system diseases. We are exploring the application of our PMO platform technology in various diseases.

We believe we have developed proprietary state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of EXONDYS 51 for commercial use and of our product candidates to support clinical development as well as commercialization. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support commercial production of EXONDYS 51 and production of certain of our product candidates and their components. We currently do not have any of our own internal mid-to-large scale manufacturing capabilities to support EXONDYS 51 or our product candidates.

The basis of our novel RNA-targeted therapeutics is the PMO. Our next generation PMO-based chemistries include PMO-X®, PMO*plus*® and PPMO. PMO and PMO-based compounds are highly resistant to degradation by enzymes, potentially enabling robust and sustained biological activity. In contrast to other RNA-targeted therapeutics, which are usually designed to down-regulate protein expression, our technologies are designed to selectively up-regulate or down-regulate protein expression, and more importantly, create novel proteins. PMO and PMO-based compounds have demonstrated inhibition of messenger RNA ("mRNA") translation and alteration of pre-mRNA splicing. PMO and PMO-based compounds have the potential to reduce off-target effects, such as the immune stimulation often observed with ribose-based RNA technologies. We believe that our highly differentiated, novel, proprietary and innovative RNA-targeted PMO-based platforms may represent a significant improvement over other RNA-targeted technologies. In addition, PMO and PMO-based compounds are highly adaptable molecules: with minor structural modifications, they can potentially be rapidly designed to target specific tissues, genetic sequences, or pathogens, and therefore, we believe they could potentially be applied to treat a broad spectrum of diseases.

We have not generated any revenue from product sales through September 30, 2016, and we may never generate substantial revenue from product sales from the commercialization of EXONDYS 51 or our pipeline of product candidates. Even if we achieve substantial revenue from product sales, we are likely to continue to incur operating losses in the near term.

As of September 30, 2016, we had approximately $406.6 million of cash, cash equivalents and restricted cash and investments, consisting of $395.1 million of cash and cash equivalents and $11.5 million of restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months. As of December 31, 2014, we had completed all development activities under our agreements with the DoD. We are currently open to possibilities for funding, collaboration and other avenues to support further development of these Ebola, Marburg and influenza product candidates. Without funding from the U.S. government, we likely will limit infectious disease research and development efforts, though we may pursue additional cash resources through public or private financings, seek additional government funding and establish collaborations with or license our technology to other companies.

The likelihood of our long-term success must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new pharmaceutical products, competitive factors in the marketplace and the complex regulatory environment in which we operate. We may never achieve significant revenue or profitable operations from the commercialization of EXONDYS 51 or any of our product candidates.

Key Financial Metrics

Revenues

Product revenue. We recognize product revenue when there is persuasive evidence of an arrangement, delivery has occurred, price to the customer is fixed or determinable and collectability is reasonably assured. Revenue from product sales will be recognized when title and risk of loss have passed to the customer. Product revenue will be recorded net of applicable reserves for discounts and allowances.

Revenue from Research Contracts and Other Grants. We recognize revenue from research contracts and other grants during the period in which the related expenses are incurred and present such revenue and related expenses on a gross basis in the unaudited

condensed consolidated financial statements. Government contracts are subject to government audits, which may result in catch-up adjustments.

If a technology, right, product or service is separate from and independent of our performance under other elements of an arrangement, we defer recognition of non-refundable up-front fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, such up-front fees are deferred and recognized over the period of continuing involvement. As of September 30, 2016, we had deferred revenue of $3.3 million, which represents up-front fees which we may recognize as revenue upon settlement of certain obligations.

Expenses

Research and Development. Research and development expenses consist of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, clinical trials and manufacturing activities.

Direct research and development expenses associated with our programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants and other external services, such as data management and statistical analysis support, and materials and supplies used in support of clinical programs. Indirect costs of our clinical programs include salaries, stock-based compensation and allocation of our facility costs.

Future research and development expenses may increase as our internal projects, such as those for our DMD product and product candidates, enter or proceed through additional clinical development. We are currently conducting various clinical trials for EXONDYS 51, including a confirmatory trial in the U.S. We completed Part I and are conducting Part II of a Phase I/IIa clinical trial for an exon 53 skipping product candidate in the E.U. We have also initiated a dose-ranging study for our exon 45 skipping product candidate in the U.S. We have begun enrolling in the U.S. and are also planning to initiate enrollment in the E.U. for a placebo-controlled confirmatory study with product candidates designed to skip exons 45 and 53. The remainder of our research and development programs are in various stages of research and preclinical development. However, our current research and development efforts may not result in additional approved products. Product candidates that appear promising at early stages of development may not reach the market for a variety of reasons. Similarly, any of our product candidates may be found to be unsafe or ineffective during clinical trials, may have clinical trials that take longer to complete than anticipated, may fail to receive necessary regulatory approvals, or may prove impracticable to manufacture in commercial quantities at reasonable cost and with acceptable quality.

As a result of these uncertainties and the other risks inherent in the drug development process, we cannot determine the duration or completion costs of current or future clinical stages of any of our product candidates. Similarly, we cannot determine when, if, or to what extent we may generate substantial revenue from the commercialization of EXONDYS 51 or any of our other product candidates. The time frame for development of any product candidate, associated development costs and the probability of regulatory and commercial success vary widely.

General and Administrative. General and administrative expenses consist principally of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, and human resource, commercial and other general and administrative functions. Other general and administrative expenses include an allocation of our facility costs and professional fees for legal, consulting and accounting services.

Interest (Expense) Income and Other, Net. Interest (expense) income and other, net, primarily consists of interest expense, interest income on our cash, cash equivalents and restricted investments and rental income. Our cash equivalents and investments consist of money market investments and certificates of deposit. Interest expense includes interest incurred on our senior secured term loan and our mortgage loans related to our Corvallis, Oregon property, a substantial portion of which has been leased to a third party since November 2011. Rental income is from leasing excess space in some of our facilities.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations is based upon our unaudited condensed consolidated financial statements included elsewhere in this report. The preparation of our unaudited condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and related disclosure of contingent assets and liabilities for the periods presented. Some of these judgments can be subjective and complex and, consequently, actual results may differ from these estimates. For any given individual estimate or assumption we make, there may also be other estimates or assumptions that are reasonable. We believe that the estimates and judgments upon which we rely are reasonable based upon historical experience and information available to us at the time when we make these estimates and judgments. To the extent there are material

differences between these estimates and actual results, our unaudited condensed consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates.

The policies that we believe are the most critical to aid the understanding of our financial results include:

- revenue recognition;
- research and development expense;
- stock-based compensation; and
- income taxes.

There have been no material changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2015.

Results of Operations for the Three and Nine Months Ended September 30, 2016 and 2015

The following tables set forth selected consolidated statements of operations data for each of the periods indicated:

	For the Three Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues	$ —	$ —	$ —	NA
Operating expenses:				
Research and development	34,349	36,673	(2,324)	(6)%
General and administrative	22,184	15,090	7,094	47%
Total operating expenses	56,533	51,763	4,770	9%
Operating loss	(56,533)	(51,763)	(4,770)	9%
Other loss:				
Interest expense and other, net	(209)	(176)	(33)	19%
Net loss	$ (56,742)	$ (51,939)	$ (4,803)	9%
Net loss per share — basic and diluted	$ (1.18)	$ (1.25)	$ 0.07	(6)%

	For the Nine Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues	$ —	$ —	$ —	NA
Operating expenses:				
Research and development	117,523	105,018	12,505	12%
General and administrative	60,812	50,714	10,098	20%
Total operating expenses	178,335	155,732	22,603	15%
Operating loss	(178,335)	(155,732)	(22,603)	15%
Other income (loss):				
Interest (expense) income and other, net	(478)	383	(861)	(225)%
Net loss	$ (178,813)	$ (155,349)	$ (23,464)	15%
Net loss per share — basic and diluted	$ (3.83)	$ (3.75)	$ (0.08)	2%

Revenues

As of December 31, 2014, we had completed all development activities related to our contracts with the U.S. government. Therefore, no revenue was recognized for the three and nine months ended September 30, 2016 or 2015 in connection with such contracts. The majority of the revenue under our U.S. government contracts has been recognized as of September 30, 2016 and only revenue for contract finalization, if any, is expected in the future.

We have not generated any revenue from product sales through September 30, 2016, and we may never generate substantial revenue from product sales from the commercialization of EXONDYS 51 or our pipeline of product candidates.

Research and Development Expenses

Our research and development expenses represent a substantial percentage of our total operating expenses, which primarily consist of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, clinical trials and manufacturing activities. We do not maintain or evaluate and, therefore, do not allocate, internal research and development costs on a project-by-project basis. As a result, a significant portion of our research and development expenses, including salaries, stock-based compensation and allocation of our facility costs, are not tracked by project, as the costs may benefit multiple projects. The following tables summarize research and development expenses by project for each of the periods indicated:

	For the Three Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands)		$	%
EXONDYS 51	$ 17,966	$ 18,853	$ (887)	(5)%
Exon 53	2,544	1,276	1,268	99%
Exon 45	1,837	1,181	656	56%
Other projects	157	173	(16)	(9)%
Internal research and development expenses	11,845	15,190	(3,345)	(22)%
Total research and development expenses	$ 34,349	$ 36,673	$ (2,324)	(6)%

	For the Nine Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands)		$	%
EXONDYS 51	$ 64,337	$ 52,353	$ 11,984	23%
Exon 53	7,584	3,446	4,138	120%
Exon 45	4,302	5,130	(828)	(16)%
Other projects	1,222	1,218	4	0%
Internal research and development expenses	40,078	42,871	(2,793)	(7)%
Total research and development expenses	$ 117,523	$ 105,018	$ 12,505	12%

The following tables summarize research and development expenses by category for each of the periods indicated:

	For the Three Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 20,773	$ 20,801	$ (28)	(0)%
Compensation and other personnel expenses	5,477	6,351	(874)	(14)%
Stock-based compensation	2,674	2,631	43	2%
Facility-related expenses	1,645	2,395	(750)	(31)%
Professional services	1,718	2,067	(349)	(17)%
Preclinical expenses	612	731	(119)	(16)%
Research and other	1,450	1,697	(247)	(15)%
Total research and development expenses	$ 34,349	$ 36,673	$ (2,324)	(6)%

	For the Nine Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 65,681	$ 57,477	$ 8,204	14%
Compensation and other personnel expenses	18,116	18,488	(372)	(2)%
Up-front license payment to UWA	7,000	—	7,000	NA
Stock-based compensation	7,527	7,639	(112)	(1)%
Facility-related expenses	5,736	7,303	(1,567)	(21)%
Professional services	5,757	5,888	(131)	(2)%
Preclinical expenses	2,583	3,239	(656)	(20)%
Research and other	5,123	4,984	139	3%
Total research and development expenses	$ 117,523	$ 105,018	$ 12,505	12%

Research and development expenses for the three months ended September 30, 2016 decreased by $2.3 million, or 6%, compared with the three months ended September 30, 2015. The decrease was primarily due to decreases of $0.9 million in compensation and other personnel expenses and $0.8 million in facility-related expenses.

Research and development expenses for the nine months ended September 30, 2016 increased by $12.5 million, or 12%, compared with the nine months ended September 30, 2015. The increase was primarily due to an increase of $8.2 million in clinical and manufacturing expenses driven by increased enrollment in our ongoing clinical trials and a $7.0 million up-front license payment to the University of Western Australia ("UWA") partially offset by a decrease of $1.6 million in facility-related expenses.

General and Administrative Expenses

The following tables summarize general and administrative expenses by category for each of the periods indicated:

	For the Three Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands)		$	%
Compensation and other personnel expenses	$ 8,238	$ 4,160	$ 4,078	98%
Stock-based compensation	6,899	2,897	4,002	138%
Professional services	4,283	6,223	(1,940)	(31)%
Facility-related expenses	1,291	815	476	58%
Other	1,473	995	478	48%
Total general and administrative expenses	$ 22,184	$ 15,090	$ 7,094	47%

	For the Nine Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands)		$	%
Compensation and other personnel expenses	$ 22,947	$ 11,289	$ 11,658	103%
Stock-based compensation	15,566	9,417	6,149	65%
Professional services	13,894	14,477	(583)	(4)%
Estimated severance expenses	—	9,182	(9,182)	(100)%
Facility-related expenses	3,385	2,624	761	29%
Other	5,020	3,725	1,295	35%
Total general and administrative expenses	$ 60,812	$ 50,714	$ 10,098	20%

General and administrative expenses for the three months ended September 30, 2016 increased by $7.1 million, or 47%, compared with the three months ended September 30, 2015. This was primarily due to increases of $4.1 million in compensation and other personnel expenses primarily driven by an increase in commercial headcount, $4.0 million in stock-based compensation primarily due to the FDA approval of EXONDYS 51 which triggered certain performance grants to become eligible for vesting, and $0.5 million in facility-related expenses. The increase was partially offset by a decrease of $1.9 million in professional services primarily due to the ramp-down of certain external commercial activities to assist in the product launch of EXONDYS 51.

General and administrative expenses for the nine months ended September 30, 2016 increased by $10.1 million, or 20%, compared with the nine months ended September 30, 2015. This was primarily due to increases of $11.7 million in compensation and other personnel expenses primarily driven by an increase in commercial headcount, $6.1 million in stock-based compensation primarily driven by an increase in headcount as well as the FDA approval of EXONDYS 51 which triggered certain performance grants to become eligible for vesting, and $0.8 million in facility-related expenses. The increase was partially offset by a decrease in severance expense of $9.2 million as a result of the resignation of our former CEO in March 2015.

Interest (Expense) Income and Other, Net

For the three months ended September 30, 2016, interest expense and other, net was relatively flat compared with the three months ended September 30, 2015.

For the nine months ended September 30, 2016, interest expense and other, net was approximately $0.5 million. For the nine months ended September 30, 2015, interest income and other, net was approximately $0.4 million. The unfavorable change was primarily driven by interest expense incurred in connection with the $20.0 million senior secured term loan.

Liquidity and Capital Resources

The following table summarizes our financial condition for each of the periods indicated:

	As of September 30, 2016	As of December 31, 2015	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 395,140	$ 80,304	$ 314,836	392%
Short-term investments	—	112,187	(112,187)	(100)%
Restricted cash and investments	11,479	11,478	1	0%
Total cash, cash equivalents and investments	$ 406,619	$ 203,969	$ 202,650	99%
Borrowings:				
Long-term debt	$ 18,598	$ 20,905	$ (2,307)	(11)%
Notes payable	—	2,493	(2,493)	(100)%
Total borrowings	$ 18,598	$ 23,398	$ (4,800)	(21)%
Working capital				
Current assets	$ 434,744	$ 224,543	$ 210,201	94%
Current liabilities	64,933	62,294	2,639	4%
Total working capital	$ 369,811	$ 162,249	$ 207,562	128%

Our principal sources of liquidity are from both equity and debt financings. Our principal uses of cash are research and development expenses, general and administrative expenses, capital expenditures and other working capital requirements.

Our future expenditures and capital requirements may be substantial and will depend on many factors, including but not limited to the following:

- the timing and costs associated with commercialization of EXONDYS 51;
- the timing and costs of building out our manufacturing capabilities;
- the timing of advanced payments related to our future inventory commitments;
- the timing and costs associated with our clinical trials and preclinical studies; and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

Our cash requirements are expected to continue to increase as we advance our research, development and commercialization programs and our business development efforts, and we may seek additional financing primarily from, but not limited to, the sale and issuance of equity, debt securities or the licensing or sale of our technology. Financing may not be available when and as needed or, if available, financings may not be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we

require, our business and results of operations will be negatively impacted. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Nine Months Ended September 30,			
	2016	2015	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (164,587)	$ (118,193)	$ (46,394)	39%
Investing activities	108,581	78,229	30,352	39%
Financing activities	370,842	22,232	348,610	1,568%
Increase (decrease) in cash and cash equivalents	$ 314,836	$ (17,732)	$ 332,568	(1,876)%

Operating Activities. The increase in cash used in operating activities of $46.4 million for the nine months ended September 30, 2016 compared with the nine months ended September 30, 2015 was primarily due to an increase of $23.5 million in net loss primarily driven by increases in research and development and general and administrative expenses, a decrease of $2.6 million in non-cash adjustments and $20.4 million of unfavorable changes in operating assets and liabilities.

Investing Activities. The cash provided by investing activities increased by $30.4 million for the nine months ended September 30, 2016 compared with the nine months ended September 30, 2015. This was primarily driven by the purchases of $49.6 million in available-for-sale securities and $10.7 million in restricted investments during the nine months ended September 30, 2015 compared to no purchases of available-for-sale securities or restricted investments for the comparable period in 2016. This was partially offset by a decrease of $29.8 million from the sale and maturity of available-for-sale securities.

Financing Activities. The increase in cash provided by financing activities of $348.6 million for the nine months ended September 30, 2016 compared with the nine months ended September 30, 2015 was primarily driven by $365.0 million in proceeds from the issuance of approximately 5.8 million shares and 2.1 million shares of common stock at an offering price of $59.75 and $17.84 per share in September 2016 and June 2016, respectively, and an increase of $5.8 million from option exercises and Employee Stock Purchase Program purchases. This was partially offset by $5.1 million in repayment of debt in connection with the promissory note related to the May 2014 acquisition of our Andover, Massachusetts facility and the senior secured term loan taken out in June 2015.

Milestone Obligations

As of September 30, 2016, we were obligated to make up to $91.5 million of future development, up-front royalty and sales milestone payments associated with certain of our collaboration and license agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or sales milestones. For the three and nine months ended September 30, 2016, we recorded $0.3 million and $7.3 million, respectively, relating to development milestone and up-front payments to UWA in connection with the license agreement and its first amendment as research and development expense in the unaudited condensed consolidated statement of operations and comprehensive loss. Additionally, corresponding to the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement, we recorded a $1.0 million sales milestone payment to UWA as an intangible asset in the unaudited condensed consolidated balance sheets as of September 30, 2016. Because the achievement of all other milestones had not occurred as of September 30, 2016, such contingencies have not been recorded in our financial statements.

Off-Balance Sheet Arrangements

During the periods presented, we did not have any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as structured finance or special purpose entities, which would have been established for the purpose of facilitating off-balance sheet arrangements or for another contractually narrow or limited purpose.

Recent Accounting Pronouncements

For additional information, please read *Note 2, Significant Accounting Policies and Recent Accounting Pronouncements* of the unaudited condensed consolidated financial statements contained in Part I, Item 1 of this report, Form 10-Q for the quarterly period ended September 30, 2016.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, government and government agency bonds and high-grade corporate bonds with maturities of three years or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of September 30, 2016, we had approximately $406.6 million of cash, cash equivalents and restricted investments, comprised of $395.1 million of cash and cash equivalents and $11.5 million of restricted cash and investments. As of September 30, 2016, our cash equivalents consist of money market investments whose fair value is not subject to change as a result of potential changes in market interest rates and we don't have other investments that are sensitive to change in market interest rate.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this Quarterly Report on Form 10-Q for the period ended September 30, 2016, under the supervision and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934 (the "Exchange Act"). The purpose of this evaluation was to determine whether as of the evaluation date our disclosure controls and procedures were effective to provide reasonable assurance that the information we are required to disclose in our filings with the SEC under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and (ii) is accumulated and communicated to our management, including our CEO and our CFO, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of September 30, 2016, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended September 30, 2016, there were no changes in the Company's internal controls over financial reporting that have materially affected or are reasonably likely to materially affect the Company's internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

For material legal proceedings, please read *Note 12, Commitments and Contingencies - Litigation* to our unaudited condensed consolidated financial statements included in this report.

Item 1A. Risk Factors.

Factors That Could Affect Future Results

Set forth below and elsewhere in this report and in other documents we file with the SEC, including the Annual Report on Form 10-K for the year ended December 31, 2015, are descriptions of risks and uncertainties that could cause actual results to differ materially from the results contemplated by the forward-looking statements contained in this report. Because of the following factors, as well as other variables affecting our operating results, past financial performance should not be considered a reliable indicator of future performance and investors should not use historical trends to anticipate results or trends in future periods. The risks and uncertainties described below are not the only ones facing us. Other events that we do not currently anticipate or that we currently deem immaterial also affect our results of operations and financial condition.

Risks Related to Our Business

We are highly dependent on the commercial success of EXONDYS 51 in the U.S.; we may not be able to meet expectations with respect to EXONDYS 51 sales or attain profitability and positive cash-flow from operations.

On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51 as a therapeutic treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is commercially available. The commercial success of EXONDYS 51 will depend on a number of factors, including:

- the effectiveness of our sales, managed markets and marketing efforts;

- FDA-mandated package insert requirements and the time it would take us to comply with any related FDA post-marketing requirements;
- demonstration and/or confirmation of clinical efficacy and safety and acceptance of the same by the medical community;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;
- whether EXONDYS 51 can consistently be manufactured in commercial quantities and at acceptable costs;
- the cost-effectiveness of the product;
- adequate reimbursement by third parties, including government payers, managed care organizations and private health insurers;
- our ability to comply with the FDA requirements, and achieve the required clinical endpoints in the studies included in the EXONDYS 51 approval letter including our ability to successfully conduct and achieve the endpoints in the two-year post-approval study required by the FDA to verify EXONDYS 51's clinical benefit;
- the need for, and success of, other confirmatory trials and post-marketing requirements;
- the development or commercialization of competing products or therapies for the treatment of DMD, or its symptoms;
- marketing and distribution support for EXONDYS 51;
- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;
- the actual market-size for EXONDYS 51, which may be different than expected;
- the sufficiency of our drug supply to meet commercial and clinical demands which could be negatively impacted if our projections on the potential number of amenable patients and their average weight are inaccurate, we are subject to unanticipated regulatory requirements that increase our drug supply needs, our current drug supply is destroyed or negatively impacted at our manufacturing sites, storage sites or in transit, or it takes longer than we project for the number of patients we anticipate to get on EXONDYS 51 and any significant portion of our EXONDYS 51 supply expires before we are able to sell it; and
- our ability to obtain regulatory approvals to commercialize EXONDYS 51 in markets outside of the U.S.

We may experience significant fluctuations in sales of EXONDYS 51 from period to period and, ultimately, we may never generate sufficient revenues from EXONDYS 51 to reach or maintain profitability or sustain our anticipated levels of operations.

EXONDYS 51 may cause undesirable side effects or have other properties that could limit its commercial potential.

If we or others identify previously unknown side effects or if known side effects are more frequent or severe than in the past, then:

- sales of EXONDYS 51 may be modest;
- regulatory approvals for EXONDYS 51 may be restricted or withdrawn;
- we may decide to, or be required to, send product warning letters or field alerts to physicians, pharmacists and hospitals;
- additional non-clinical or clinical studies, changes in labeling or changes to manufacturing processes, specifications and/or facilities may be required; and
- government investigations or lawsuits, including class action suits, may be brought against us.

Any of the above occurrences would harm or prevent sales of EXONDYS 51, increase our expenses and impair our ability to successfully commercialize EXONDYS 51. Furthermore, once EXONDYS 51 is commercially available, it may be used in a wider population and in a less rigorously controlled environment than in clinical studies. As a result, regulatory authorities, healthcare practitioners, third-party payers or patients may perceive or conclude that the use of EXONDYS 51 is associated with previously unknown serious adverse effects, undermining our commercialization efforts.

We currently rely on third parties to manufacture EXONDYS 51 and to produce our product candidates; our dependence on these parties, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet commercial or clinical product demand may impair the commercialization of EXONDYS 51 and the research and development programs and potential commercialization of our product candidates.

We currently do not have the internal ability to undertake the manufacturing process for EXONDYS 51 or our product candidates in the quantities needed to meet commercial demand for EXONDYS 51, or to conduct our research and development programs and conduct clinical trials for our product candidates. Therefore, we rely on and expect to continue relying on for the foreseeable future, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), drug substance ("API") and drug product, as well as to perform additional steps in the manufacturing process, such as the filling and labeling of vials and storage of EXONDYS 51 and our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of EXONDYS 51 and our product candidates, which creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require. Any interruption of the development or operation of those facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available EXONDYS 51, product candidates or materials.

If these third parties were to cease providing quality manufacturing and related services to us, and we are not able to engage appropriate replacements in a timely manner, our ability to manufacture EXONDYS 51 or our product candidates in sufficient quality and quantity required for commercial use of EXONDYS 51 and/ or for planned pre-clinical testing, clinical trials and potential commercial use of our product candidates would be adversely affected.

We have, through our third-party manufacturers, produced or are in the process of producing clinical and commercial supply of our product candidates and EXONDYS 51, respectively, based on our current understanding of market demands and our needs for our research and development efforts and clinical trials. In light of the limited number of third parties with the expertise to produce EXONDYS 51 and our product candidates, the lead time needed to manufacture them, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of EXONDYS 51 to meet demands that exceed our commercial assumptions or to provide adequate supply of our product candidates to meet demands that exceed our clinical assumptions. Furthermore, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for EXONDYS 51 and our product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which could impact our ability to execute our business plans on expected or required timelines in connection with the commercialization of EXONDYS 51 and the continued development of our product candidates. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

The third parties we use in the manufacturing process for EXONDYS 51 and our product candidates may fail to comply with cGMP regulations.

Our contract manufacturers are required to produce our materials, APIs and drug products under current Good Manufacturing Practice regulations ("cGMP"). We and our contract manufacturers are subject to periodic inspections by the FDA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. We do not have control over a third-party manufacturer's compliance with these regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of EXONDYS 51 and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. The failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in product recalls, patient injury or death. If our contract manufacturers fail to adhere to applicable cGMP and other applicable government regulations, or experience manufacturing problems, we will suffer significant consequences, including product seizures or recalls, postponement or cancellation of clinical trials, loss or delay of product approval, fines and sanctions, loss of revenue, termination of the development of a product candidate, reputational damage, shipment delays, inventory shortages, inventory write-offs and other product-related charges and increased manufacturing costs. If we experience any of these results, we may not be able to successfully commercialize EXONDYS 51.

We may not be able to successfully scale up manufacturing of EXONDYS 51 or our product candidates in sufficient quality and quantity or within sufficient timelines, or be able to secure ownership of intellectual property rights developed in this process, which could negatively impact our commercialization of EXONDYS 51 and or the development of our product candidates.

We are working to increase manufacturing capacity and scale up production of some of the components of our drug products. During the remainder of 2016, our focus remains on (i) achieving larger-scale manufacturing capacity for EXONDYS 51 throughout the manufacturing supply chain and (ii) continuing to increase material and API production capacity to provide the anticipated amounts of drug product needed for our planned studies for our product candidates. We may not be able to successfully increase manufacturing capacity or scale up the production of materials, APIs and drug products, whether in collaboration with third-party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory requirements, in a cost-effective manner, in a time frame required to meet our timeline for commercialization, clinical trials and other business plans, or at all. Compliance with cGMP requirements and other quality issues may arise during our efforts to increase manufacturing capacity and scale up production with our current or any new contract manufacturers. These issues may arise in connection with the underlying materials, the inherent properties of EXONDYS 51 or a product candidate, EXONDYS 51 or a product candidate in combination with other components added during the manufacturing and packaging process or during shipping and storage of the APIs or finished drug product. In addition, in order to release EXONDYS 51 for commercial use and demonstrate stability of product candidates for use in late stage clinical trials (and any subsequent drug products for commercial use), our manufacturing processes and analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of EXONDYS 51 or our product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, the commercial availability of EXONDYS 51 and the continued development and/or regulatory approval of our product candidates may be delayed, which could significantly harm our business.

During work with our third-party manufacturers to increase manufacturing capacity and scale up production, it is possible that they could make proprietary improvements in the manufacturing and scale-up processes for EXONDYS 51 or our product candidates. We may not own or be able to secure ownership of such improvements or may have to share the intellectual property rights to those improvements. Additionally, it is possible that we will need additional processes, technologies and validation studies, which could be costly and which we may not be able to develop or acquire from third parties. Any failure to secure the intellectual rights required for the manufacturing process needed for large-scale clinical trials or commercialization of EXONDYS 51 or the continued development of our product candidates could cause significant delays in our business plans or otherwise negatively impact the commercialization of EXONDYS 51 or the continued development of our product candidates.

If we are unable to maintain our agreements with third parties to distribute EXONDYS 51 to patients, our results of operations and business could be adversely affected.

We will rely on third parties to commercially distribute EXONDYS 51 to patients. We have contracted with a third-party logistics company to warehouse EXONDYS 51 and with specialty pharmacies to sell and distribute it to patients. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications for complex or chronic conditions that require a high level of patient education and ongoing management. We are also planning to contract with a third-party call center to help us with some or all of the following: coordinate prescription intake and distribution, reimbursement adjudication, patient financial support, and ongoing compliance support. This distribution network will require significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from EXONDYS 51. If we are unable to effectively manage the distribution process, the commercial launch and sales of EXONDYS 51, as well as any future products we may commercialize, could be delayed or severely compromised and our results of operations may be harmed.

In addition, the use of specialty pharmacies and a call center involves certain risks, including, but not limited to, risks that these organizations will:

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using EXONDYS 51 or serious adverse events and/or product complaints regarding EXONDYS 51;
- not effectively sell or support EXONDYS 51;
- reduce or discontinue their efforts to sell or support EXONDYS 51;
- not devote the resources necessary to sell EXONDYS 51 in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

Any such events may result in decreased product sales and lower product revenue, which would harm our results of operations and business.

If we are unable to successfully maintain and further develop internal commercialization capabilities, sales of EXONDYS 51 may be negatively impacted.

We have hired a commercial team and put in the organizational infrastructure we believe we need for a successful commercial launch of EXONDYS 51. We will need to commit significant time and financial and managerial resources to maintain and further develop our marketing and sales force to ensure they have the technical expertise required to address any challenges we may face with the commercialization of EXONDYS 51. Factors that may inhibit our efforts to maintain and develop our commercialization capabilities include:

- an inability to retain an adequate number of effective commercial personnel;
- an inability to train sales personnel, who may have limited experience with our company or EXONDYS 51, to deliver a consistent message regarding EXONDYS 51 and be effective in convincing physicians to prescribe EXONDYS 51;
- an inability to equip sales personnel with effective materials, including medical and sales literature to help them educate physicians and our healthcare providers regarding EXONDYS 51 and its proper administration;
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization.

If we are not successful in establishing and maintaining an effective sales and marketing infrastructure, we will have difficulty commercializing EXONDYS 51, which would adversely affect our business and financial condition.

Even though EXONDYS 51 has been approved by the FDA as a treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping, it faces future post-approval development and regulatory requirements, which will present additional challenges.

On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51 as a therapeutic treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. This indication is based on an increase in dystrophin in skeletal muscles observed in some patients treated with EXONDYS 51. EXONDYS 51 will be subject to ongoing FDA requirements governing the labeling, packaging, storage, advertising, promotion, recordkeeping and submission of safety, efficacy and other post-market information.

Under the accelerated approval provisions, the FDA is requiring that the Company complete various post-approval requirements including conducting a clinical trial to verify the drug's clinical benefit. If the trial fails to verify clinical benefit, the FDA may initiate proceedings to withdraw approval of the drug. These post-approval requirements could impose significant burdens and costs on us. Failure to meet post-approval commitments, including obtaining positive safety and efficacy data from our confirmatory studies for EXONDYS 51, would lead to negative regulatory action from the FDA, which could include withdrawal of regulatory approval of EXONDYS 51.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. If we or a regulatory agency discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with a facility where the product is manufactured, a regulatory agency may impose restrictions on that product or the manufacturer, including requiring implementation of a risk evaluation and mitigation strategy program, withdrawal of the product from the market or suspension of manufacturing. If we or the manufacturing facilities for EXONDYS 51 fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications submitted by us;
- impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products or require us to initiate a product recall.

Even though EXONDYS 51 has been approved for marketing in the U.S., we may never receive approval to commercialize EXONDYS 51 outside of the U.S.

In the future, we may seek to commercialize EXONDYS 51 in foreign countries outside of the U.S. In order to market any products outside of the U.S., we must comply with numerous and varying regulatory requirements of other jurisdictions regarding safety and efficacy. Approval procedures vary among jurisdictions and can involve product testing and administrative review periods different from, and greater than, those in the U.S.

Regulatory approval in one jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory processes in others. Failure to obtain regulatory approvals in other jurisdictions or any delay or setback in obtaining such approvals could adversely affect our business and financial condition.

EXONDYS 51 may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our potential profitability and future business prospects.

The commercial success of EXONDYS 51, particularly in the near term in the U.S., depends upon its level of market adoption by patients, payors and healthcare providers. If EXONDYS 51 does not achieve an adequate level of market adoption for any reason, our potential profitability and our future business prospects will be severely adversely impacted. The degree of market acceptance of EXONDYS 51 depends on a number of factors, including:

- our ability to demonstrate to the medical community, including specialists who may purchase or prescribe EXONDYS 51, the clinical efficacy and safety of EXONDYS 51 as the prescription product of choice DMD amenable to exon-51 skipping in the U.S.;
- the effectiveness of our sales and marketing organizations and distribution networks;
- the ability of patients or providers to be adequately reimbursed for EXONDYS 51 in a timely manner from government and private payors; and
- the actual and perceived efficacy and safety profile of EXONDYS 51, particularly if unanticipated adverse events related to EXONDYS 51 treatment arise and create safety concerns among potential patients or prescribers.

The patient population suffering from DMD, and in particular those with mutations amenable to exon-51 skipping, is small and has not been established with precision. If the actual number of patients is smaller than we estimate, our revenue and ability to achieve profitability may be adversely affected.

DMD is a fatal genetic neuromuscular disorder affecting an estimated one in approximately every 3,500-5,000 males born worldwide, of which up to 13% are estimated to be amenable to exon-51 skipping. Our estimate of the size of the patient population is based on published studies as well as internal analyses. If the results of these studies or our analysis of them do not accurately reflect the number of patients with DMD, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to obtain and maintain profitability. Since EXONDYS 51 targets a small patient population, the per-patient drug pricing must be high in order to recover our development and manufacturing costs, fund adequate patient support programs, fund additional research and achieve profitability. We may be unable to maintain or obtain sufficient sales volumes at a price high enough to justify our product development efforts and our sales, marketing and manufacturing expenses.

We have been granted orphan drug designations in the U.S. and in the E.U. for certain of our product candidates, however, there can be no guarantee that we will maintain orphan status for these product candidates nor that we will receive orphan drug approval and prevent third parties from developing and commercializing products that are competitive to these product candidates in the absence of other barriers to entry.

To date, in addition to the orphan drug exclusivity described above for EXONDYS 51, we have been granted orphan drug designation by the FDA under the Orphan Drug Act for an additional product candidate in DMD, AVI-7537 for the treatment of Ebola virus and AVI-7288 for the treatment of the Marburg virus.

We also have been granted orphan medicinal product designations in the European Union ("E.U.") for two of our product candidates in DMD (including EXONDYS 51). Product candidates granted orphan status in Europe can be provided with up to ten years of marketing exclusivity, meaning that another application for marketing authorization of a later, similar medicinal product for the same therapeutic indication will generally not be approved in Europe during that time period. Although we may have product candidates that obtain orphan drug exclusivity in Europe, the orphan status and associated exclusivity period may be modified for

several reasons, including a significant change to the orphan medicinal product designations or status criteria after-market authorization of the orphan product (*e.g.* , product profitability exceeds the criteria for orphan drug designation), problems with the production or supply of the orphan drug, or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial orphan drug.

As discussed above, we are not guaranteed to receive or maintain orphan status for our current or future product candidates, and if our product candidates that are granted orphan status were to lose their status as orphan drugs or the marketing exclusivity provided for them in the U.S. or the E.U., our business and results of operations could be materially adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the U.S. and the E.U. for the time periods specified above, we would not be able to exclude other companies from manufacturing and/or selling products using the same active ingredient for the same indication beyond the exclusivity period applicable to our product on the basis of orphan drug status. In addition, we cannot guarantee that another company will not receive approval to market a product candidate that is granted orphan drug status in the U.S. or the E.U. for a product candidate that has the same active ingredient or is a similar medicinal product for the same indication as any of our product candidates for which we plan to file a new drug application ("NDA") or marketing authorization application ("MAA"). If that were to happen, any pending NDA or MAA for our product candidate for that indication may not be approved until the competing company's period of exclusivity has expired in the U.S. or the E.U., as applicable. Furthermore, application of the orphan drug regulations in the U.S. and Europe is uncertain, and we cannot predict how the respective regulatory bodies will interpret and apply the regulations to our or our competitors' product candidates.

If we are unable to maintain orphan drug exclusivity for EXONDYS 51 in the U.S., we may face increased competition.

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition affecting fewer than 200,000 people in the U.S. A company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition generally receives orphan drug marketing exclusivity for that drug for a period of seven years from the date of its approval. This orphan drug exclusivity prevents the approval of another drug containing the same active ingredient and used for the same orphan indication except in very limited circumstances, based on the FDA's determination that a subsequent drug is safer, more effective or makes a major contribution to patient care, or if the orphan drug manufacturer is unable to assure that a sufficient quantity of the orphan drug is available to meet the needs of patients with the rare disease or condition. Orphan drug exclusivity may also be lost if the FDA later determines that the initial request for designation was materially defective. EXONDYS 51 was granted orphan drug exclusivity for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping, which we expect will provide the drug with orphan drug marketing exclusivity in the U.S. until September 19, 2023, seven years from the date of its approval. However, such exclusivity may not effectively protect the product from competition if the FDA determines that a subsequent drug for the same indication is safer, more effective or makes a major contribution to patient care, or if we are unable to assure the FDA that sufficient quantities of EXONDYS 51 are available to meet patient demand. In addition, orphan drug exclusivity does not prevent the FDA from approving competing drugs for the same or similar indication containing a different active ingredient. If a subsequent drug is approved for marketing for the same or similar indication, we may face increased competition, and our revenues from the sale of EXONDYS 51 will be adversely affected.

We are subject to uncertainty relating to reimbursement policies which, if not favorable for EXONDYS 51, could hinder or prevent EXONDYS 51's commercial success.

Our ability to successfully commercialize EXONDYS 51 in the U.S. will depend in part on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors. Third-party payors are increasingly challenging the effectiveness of and prices charged for medical products and services. We may not obtain adequate third-party coverage or reimbursement for EXONDYS 51, or we may be required to sell EXONDYS 51 at an unsatisfactory price.

We expect that private insurers will consider the efficacy, cost-effectiveness and safety of EXONDYS 51 in determining whether to approve reimbursement for EXONDYS 51 and at what level. Obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we do not receive approval for reimbursement of EXONDYS 51 from private insurers on a timely or satisfactory basis. Our business could also be adversely affected if private insurers, including managed care organizations, the Medicare or Medicaid programs or other reimbursing bodies or payors limit the indications for which EXONDYS 51 will be reimbursed.

In some foreign countries, particularly Canada and the countries of Europe, the pricing of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing negotiations with governmental authorities can take six to 12 months or longer after the receipt of regulatory approval and product launch. To obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our products, including EXONDYS 51, to other available therapies. Furthermore, several European countries have implemented government measures to either freeze or reduce pricing of pharmaceutical products. If reimbursement for our products is unavailable in any

country in which reimbursement is sought, limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

We expect to experience pricing pressures in connection with the sale of EXONDYS 51 and our future products due to the healthcare reforms discussed below, as well as the trend toward programs aimed at reducing healthcare costs, the increasing influence of health maintenance organizations and additional legislative proposals.

We will incur significant liability if it is determined that we are promoting any "off-label" use of EXONDYS 51.

Physicians are permitted to prescribe drug products for uses that are not described in the product's labeling and that differ from those approved by applicable regulatory agencies. Off-label uses are common across medical specialties. Although the FDA and other regulatory agencies do not regulate a physician's choice of treatments, the FDA and other regulatory agencies do prohibit advertising and promotion of off-label uses of approved drug products or promotion of an approved drug on information that is not in the final, FDA-approved label for a product and restrict communications on off-label use. Accordingly, we may not promote EXONDYS 51 in the U.S. for use in any indications other than for the treatment of DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. Additionally, we are not able to promote EXONDYS 51 based on any information excluded in the final FDA-approved label, including previously published clinical data. The FDA and other regulatory authorities actively enforce laws and regulations prohibiting promotion of a product for off-label uses and the promotion of products for which marketing approval has not been obtained. A company that is found to have improperly promoted its drug product will be subject to significant liability, including civil and administrative remedies as well as criminal sanctions.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading and non-promotional scientific exchange concerning their products. We intend to engage in medical education activities and communicate with healthcare providers in compliance with all applicable laws, regulatory guidance and industry best practices. Although we have established a compliance program and continue to enhance it to ensure that all such activities are performed in a legal and compliant manner, EXONDYS 51 is our first commercial product which could increase risk of non-compliance with our internal compliance policies and applicable rules and regulations, which could negatively impact our business.

Most of our product candidates are at an early stage of development and may never receive regulatory approval.

Other than EXONDYS 51 which the FDA approved for use in the U.S. in September 2016 and for which we plan to file an MAA by end of year with EMA, our most advanced product candidates are exon 45 and 53 skipping products. We are in the process of conducting, starting or planning various EXONDYS 51 clinical studies including studies that are required to comply with regulatory NDA and/or MAA filing requirements as well as studies we need to conduct to comply with our post-marketing FDA requirements to verify and describe clinical benefit. The exon 53-skipping product candidate, which we are working on with the SKIP-NMD consortium, is currently in the clinic in EU. The Part I dose-titration portion of this Phase I/IIa study has been completed and Part II open label portion of the study is ongoing. We have also completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. Additionally, we are enrolling patients in the U.S. and working towards initiating sites in the E.U. and Canada for a clinical trial using exon 45- and 53-skipping product candidates, which we refer to as the ESSENCE study. The remainder of our product candidates are in discovery or early stages of development. These product candidates will require significant further development, financial resources and personnel to develop into commercially viable products and obtain regulatory approval, if at all. Currently, our exon 45-skipping product candidate, the exon 53-skipping product candidate we are developing with the SKIP-NMD consortium, each for DMD, and radavirsen (formerly AVI-7100) for influenza are in active clinical development. Our other product candidates, including our anti-bacterials and AVI-7537 in Ebola/ and AVI-7288, are in discovery, pre-clinical development or inactive. Given the FDA approval of EXONDYS 51, we expect that much of our effort and many of our expenditures over the next several years will be devoted to clinical development and regulatory activities associated with EXONDYS 51 and other exon-skipping candidates as part of our larger pan-exon strategy in DMD, our infectious disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates or successfully obtain approvals needed to market them. Although EXONDYS 51 was approved under accelerated approval by the FDA in the U.S., we may be delayed in or may not be able to successfully submit an MAA to EMA that leads to an approval of EXONDYS 51 in the E.U.

Our RNA-targeted antisense technology has only been incorporated into one therapeutic commercial product and additional studies may not demonstrate safety or efficacy of our technology in other product candidates.

Our RNA-targeted platform, utilizing proprietary PMO-based technology has only been incorporated into one therapeutic commercial product to date, EXONDYS 51, however, our confirmatory trials for EXONDYS 51 must verify and describe the clinical benefits in order for EXONDYS 51 to remain approved in the U.S. All of our product candidates to date use our PMO-based

technology. Although we have conducted and are in the process of conducting clinical studies with EXONDYS 51, an exon 45-skipping product candidate and an exon 53-skipping product candidate and pre-clinical studies with our other product candidates that use our PMO-based antisense technology, additional studies may be needed to determine the safety and efficacy of our PMO-based antisense technology. In addition, nonclinical models used to evaluate the activity and toxicity of product candidate compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease. As such, there may be substantially different results observed in clinical trials from those observed in pre-clinical studies. Any failures or setbacks in developing or utilizing our PMO-based technology, including adverse effects in humans, could have a detrimental impact on our product candidate pipeline and our ability to maintain and/or enter into new corporate collaborations regarding these technologies, which would negatively affect our business and financial condition.

If there are significant delays in obtaining or we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates in a timely manner or at all, which would materially impair our ability to generate revenue and have a successful business.

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by applicable local, regional and national regulatory authorities and regulations may differ from jurisdiction to jurisdiction. In the U.S., approvals and oversight from federal (e.g., FDA), state and other regulatory authorities are required for these activities. Sale and marketing of our product candidates in the U.S. or other countries is not permitted until we obtain the required approvals from the applicable regulatory authorities. Our ability to obtain the government or regulatory approvals required to commercialize any of our product candidates in any jurisdiction, including in the U.S., cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our non-clinical, clinical, Chemistry, Manufacturing and Controls ("CMC") and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for submissions of a marketing application or approvals. The FDA could disagree with our beliefs, interpretations and conclusions regarding data we provide in connection with an NDA submission for one of our product candidates, and may delay, reject or refuse to file or approve any NDA submission we make or identify additional requirements for product approval in a complete response letter to be submitted upon completion, if ever. In addition, an advisory committee could determine our data are insufficient to provide a positive recommendation for approval of any NDA we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA submission, the FDA could still deny approval of our product candidates based on their review of the data or other factors.

- The regulatory approval process for product candidates targeting orphan diseases, such as DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, and novel endpoints, such as natural history data and dystrophin measures, is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. We cannot be sure that any of our product candidates will qualify for accelerated approval under Food and Drug Administration Safety and Innovation Act or any other expedited development, review and approval programs, or that, if a drug does qualify, that the product candidates will be approved, will be accepted as part of any such program or that the review time will be shorter than a standard review. As a result of uncertainty in the approval process, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned investigational new drug applications ("INDs") and NDAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (e.g., additional patient muscle biopsies and dystrophin analyses), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, an advisory committee or regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could negatively impact the review of our NDA or result in a decision by the Company not to proceed with the development of a product candidate or an NDA submission for a product candidate based on feedback from regulators.

- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates. Any failure on our part to respond to these requirements in a timely and satisfactory manner could significantly delay or negatively impact confirmatory study timelines and/or the development plans we have for the exon 53- and exon 45-skipping or other product candidates. Responding to requests from regulators and meeting requirements for clinical studies, submissions, filings, advisory committees and approvals may require substantial personnel, financial or other resources, which, as a small pre-commercial biopharmaceutical company, we may not be able to obtain in a timely manner or at all. In addition, our ability to respond to requests from regulatory authorities that involve our agents, third-party vendors and associates may be complicated by our own limitations and those of the parties we work with. It may be difficult or impossible for us to conform to regulatory guidance or successfully execute our product development plans in response to regulatory guidance, including guidance related to clinical trial design and the timing of regulatory decisions with respect to any NDA submissions.

Due to the above factors, among others, our product candidates could take a significantly longer time to gain regulatory approval than we expect, or may never gain regulatory approval, which would delay or eliminate any potential commercialization or product revenue for us and result in a material adverse effect on the Company that could involve changes, delays in or terminations of programs in our pipeline, delays or terminations of pre-clinical and clinical studies, and termination of contracts related to the development of our product candidates which can include significant termination costs, workforce reductions and limited ability to raise additional funds to execute company plans.

Even if we are able to comply with all regulatory requests and requirements, the delays resulting from satisfying such requests and requirements, the cost of compliance, or the effect of regulatory decisions (e.g., decisions limiting labeling and indications requested by us for a product candidate) may no longer make commercialization of a product candidate desirable for us from a business perspective, which could lead us to decide not to commercialize a product candidate.

Even after approval and commercialization of a product candidate, we would remain subject to ongoing regulatory compliance and oversight to maintain our approval. Conducting our confirmatory studies could take years to complete, could yield negative or uninterpretable results or could result in an FDA determination that the studies do not provide the safety and efficacy requirements to maintain regulatory approval. If we are not able to maintain regulatory compliance, we may be subject to civil and criminal penalties or we may not be permitted to continue marketing our products, which could have a material adverse effect on our financial condition and harm our competitive position in the market place.

Our pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical studies that the product candidate is safe and effective in humans. Ongoing and future pre-clinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. Furthermore, success in pre-clinical and early clinical trials does not ensure that the subsequent trials will be successful, nor does it predict final results of a confirmatory trial. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, then the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld. For example, we cannot provide assurances that data from any of our ongoing studies will be positive and consistent through the study periods or that the interpretation by regulators, such as the FDA, of the data we collect for our product candidates will be consistent with our interpretations.

If we fail to comply with healthcare and other regulations, we could face substantial penalties and our business, operations and financial condition could be adversely affected.

As a manufacturer of pharmaceuticals, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. We will be subject to healthcare fraud and abuse and patient privacy regulation by both the federal government and the states in which we conduct our business. The regulations include:

- federal healthcare program anti-kickback laws, which prohibit, among other things, persons from soliciting, receiving or providing remuneration, directly or indirectly, to induce either the referral of an individual, for an item or service or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;

- federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, information or claims for payment from Medicare, Medicaid or other third-party payers that are false or fraudulent, and which may apply to us for reasons including providing coding and billing advice to customers;

- the federal Health Insurance Portability and Accountability Act of 1996, which prohibits executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters and which also imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information;

- the Federal Food, Drug and Cosmetic Act, which among other things, strictly regulates drug product and medical device marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;

- federal laws that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under government healthcare programs;

- the so-called "federal sunshine" law, which requires pharmaceutical and medical device companies to monitor and report certain financial interactions with physicians and other healthcare professionals and healthcare organizations to the federal government for re-disclosure to the public; and

- state law equivalents of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payer, including commercial insurers, state transparency laws and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by federal laws, thus complicating compliance efforts.

If our operations are found to be in violation of any of the laws described above or any governmental regulations that apply to us, we will be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results.

In connection with the commercial launch of EXONDYS 51, we have initiated our compliance program and are in the process of assembling an experienced compliance team that will continue to develop a program based on industry best practices that is designed to ensure that our commercialization of EXONDYS 51 complies with all applicable laws, regulations and industry standards. As this program has not yet been tested and the requirements in this area are constantly evolving, we cannot be certain that our program will eliminate all areas of potential exposure. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against such action, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security, fraud and reporting laws may prove costly.

Healthcare reform and other governmental and private payor initiatives may have an adverse effect upon, and could prevent commercial success of EXONDYS 51 and our other product candidates.

The U.S. government and individual states are aggressively pursuing healthcare reform, as evidenced by the passing of the Patient Protection and Affordable Care Act, as modified by the Health Care and Education Reconciliation Act of 2010. These healthcare reform laws contain several cost containment measures that could adversely affect our future revenue, including, for example, increased drug rebates under Medicaid for brand name prescription drugs, extension of Medicaid rebates to Medicaid managed care plans, and extension of so-called 340B discounted pricing on pharmaceuticals sold to certain healthcare providers. Additional provisions of the healthcare reform laws that may negatively affect our future revenue and prospects for profitability include the assessment of an annual fee based on our proportionate share of sales of brand name prescription drugs to certain government programs, including Medicare and Medicaid, as well as mandatory discounts on pharmaceuticals sold to certain Medicare Part D beneficiaries. Other aspects of healthcare reform, such as expanded government enforcement authority and heightened standards that could increase compliance-related costs, could also affect our business.

In addition to government efforts in the U.S., foreign jurisdictions as well as private health insurers and managed care plans are likely to continue challenging manufacturers' ability to obtain reimbursement, as well as the level of reimbursement, for pharmaceuticals and other healthcare-related products and services. These cost-control initiatives could significantly decrease the available coverage and the price we might establish for EXONDYS 51 and our other potential products, which would have an adverse effect on our financial results.

The Food and Drug Administration Amendments Act of 2007 also provides the FDA enhanced post-marketing authority, including the authority to require post-marketing studies and clinical trials, labeling changes based on new safety information, and

compliance with risk evaluations and mitigation strategies approved by the FDA. The FDA's exercise of this authority could result in increased development-related costs following the commercial launch of EXONDYS 51, and could result in potential restrictions on the sale and/or distribution of EXONDYS 51, even in its approved indications and patient populations.

We rely on third parties to provide services in connection with our pre-clinical and clinical development programs. The inadequate performance by or loss of any of these service providers could affect our product candidate development.

Several third parties provide services in connection with our pre-clinical and clinical development programs, including in vitro and in vivo studies, assay and reagent development, immunohistochemistry, toxicology, pharmacokinetics, clinical assessments, data monitoring and management, statistical analysis and other outsourced activities. If these service providers do not adequately perform the services for which we have contracted or cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our product candidates, our development programs may be delayed.

We are winding down our expired U.S. government contract, and thus further development of our Ebola and Marburg product candidates may be limited by our ability to obtain additional funding for these programs and by the intellectual property and other rights retained by the U.S. government.

We have historically relied on U.S. government contracts and awards to fund and support certain development programs, including our Ebola and Marburg programs. The July 2010 U.S. Department of Defense ("DoD") contract providing funds for our Marburg program expired in July 2014, and the Ebola portion of the contract was previously terminated by the DoD in 2012 for convenience of the DoD. We are currently involved in contract wind-down activities and may be subject to additional government audits prior to collecting final cost reimbursements and fees owed by the government. If we are not able to complete such audits or other government requirements successfully, then the government may withhold some or all of the currently outstanding amounts owed to us. We may explore and evaluate options to continue advancing the development of our Ebola and Marburg product candidates, which may or may not include funding through U.S. government programs. As a result of government budgetary cuts, appropriations and sequestration, among other reasons, the viability of the government and its agencies as a partner for further development of our Ebola and Marburg programs, or other programs, is uncertain. The options for us to further develop product candidates that were previously developed under contracts with the U.S. government with third parties may be limited or difficult in certain respects given that, after termination or expiration of a U.S. government contract, the government has broad license rights in intellectual property developed under such contract. Therefore, the U.S. government may have the right to develop all or some parts of product candidates that we have developed under a U.S. government contract after such contract has terminated or expired.

We may not be able to successfully conduct clinical trials due to various process-related factors which could negatively impact our business plans.

The successful start and completion of any of our clinical trials within time frames consistent with our business plans is dependent on regulatory authorities and various factors, which include, but are not limited to, our ability to:

- recruit and retain employees, consultants or contractors with the required level of expertise;
- recruit and retain sufficient patients needed to conduct a clinical trial;
- enroll and retain participants, which is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, activities of patient advocacy groups, the eligibility criteria for the trial, the existence of competing clinical trials, the availability of alternative or new treatments, side effects from the therapy, lack of efficacy, personal issues and ease of participation;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the contract research organizations ("CROs") involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and IRBs, such as informed consents, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting the Company to various risks;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- manage or resolve unforeseen adverse side effects during a clinical trial;

- conduct the clinical trials in a cost-effective manner, including managing foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain Company functions during the clinical trial; and
- execute clinical trial designs and protocols approved by regulatory authorities without deficiencies.

If we are not able to manage the clinical trial process successfully, our business plans could be delayed or be rendered unfeasible for us to execute within our planned or required time frames, or at all.

We have incurred operating losses since our inception and we may not achieve or sustain profitability.

We incurred an operating loss of $178.3 million for the nine months ended September 30, 2016. Our accumulated deficit was $1.1 billion as of September 30, 2016. Although we launched EXONDYS 51 in the U.S. in September 2016, we believe that it will take us some time to attain profitability and positive cash flow from operations. Substantially all of our revenue to date has been derived from research and development contracts with the DoD, the last of which expired in July 2014. We have not yet generated any revenue from product sales and have generally incurred expenses related to research and development of our technology and product candidates, from general and administrative expenses that we have incurred while building our business infrastructure. We anticipate that our expenses will increase substantially if and/or as we:

- launch and commercialize EXONDYS 51 in the U.S.;
- establish our sales, marketing and distribution capabilities;
- continue our research, pre-clinical and clinical development of our product candidates;
- respond to and satisfy requests and requirements from regulatory authorities in connection with development and potential approval of our product candidates;
- initiate additional clinical trials for our product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- acquire or in-license other product candidates;
- maintain, expand and protect our intellectual property portfolio;
- increase manufacturing capabilities including capital expenditures related to our real estate facilities and entering into manufacturing agreements;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

As a result, we expect to continue to incur significant operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when, or if, we will become profitable.

We will need additional funds to conduct our planned research, development, manufacturing and business development efforts. If we fail to attract and manage significant capital on acceptable terms or fail to enter into strategic relationships, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We will likely require additional capital from time to time in the future in order to meet FDA post-marketing approval requirements and market and sell EXONDYS 51 as well as continue the development of product candidates in our pipeline, to expand our product portfolio and to continue or enhance our business development efforts. The actual amount of funds that we may need and the sufficiency of the capital we have or are able to raise will be determined by many factors, some of which are in our control and others that are beyond our control. The Company and the board of directors continue to assess optimization in the size and structure of the Company as well as in its strategic plans. For example, in March 2016, we announced a long-term plan to consolidate facilities within Massachusetts and closing our Corvallis, Oregon offices by end of year. Any failure on our part to strategically and successfully manage the funds we raise, with respect to factors within our control, could impact our ability to successfully commercialize EXONDYS 51 and continue developing our product candidates. Some of the factors partially or entirely outside of our control that could impact our ability to raise funds, as well as the sufficiency of funds the Company has to execute its business plans successfully, include the success of our research and development efforts, the status of our pre-clinical and clinical testing, costs and

timing relating to securing regulatory approvals and obtaining patent rights, regulatory changes, competitive and technological developments in the market, regulatory decisions, and any commercialization expenses related to any product sales, marketing, manufacturing and distribution. An unforeseen change in these factors, or others, might increase our need for additional capital.

We would expect to seek additional financing from the sale and issuance of equity or equity-linked or debt securities, and we cannot predict that financing will be available when and as we need financing or that, if available, the financing terms will be commercially reasonable. If we are unable to obtain additional financing when and if we require it, or on commercially reasonable terms, this would have a material adverse effect on our business and results of operations.

If we are able to consummate such financings, the trading price of our common stock could be adversely affected and/or the terms of such financings may adversely affect the interests of our existing stockholders. To the extent we issue additional equity securities or convertible securities, our existing stockholders could experience substantial dilution in their economic and voting rights. Additional 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. We could also be required to seek funds through arrangements with collaborators or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates, sell our Priority Review Voucher, or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

Further, we may also enter into relationships with pharmaceutical or biotechnology companies to perform research and development with respect to our technologies, research programs, conduct clinical trials or market our product candidates. Other than pre-clinical collaborations with academic or research institutions and government entities for the development of additional exon-skipping product candidates for the treatment of DMD and clinical collaboration for a product candidate for the treatment of influenza, we currently do not have a strategic relationship with a third party to perform research or development using our technologies or assist us in funding the continued development and commercialization of any of our programs or product candidates. If we were to have such a strategic relationship, such third party may require us to issue equity to such third party, 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.

Our indebtedness resulting from our credit and security agreement with MidCap Financial could adversely affect our financial condition or restrict our future operations.

On June 26, 2015, the Company entered into a credit and security agreement with MidCap Financial that provides a senior secured term loan of $20.0 million. This indebtedness could have important consequences, including:

- requiring the Company to maintain pledged cash in favor of MidCap Financial equal to but not less than the lesser of the outstanding term loans or $15.0 million;
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry;
- placing us at a competitive disadvantage compared to our competitors who have less debt or competitors with comparable debt at more favorable interest rates;
- limiting our ability to borrow additional amounts for working capital, capital expenditures, research and development efforts, acquisitions, debt service requirements, execution of our business strategy and other purposes; and
- resulting in an acceleration of the maturity of such term loans upon the occurrence of a material adverse change or another default under the credit and security agreement.

Any of these factors could materially and adversely affect our business, financial condition and results of operations.

The estimates and judgments we make, or the assumptions on which we rely, in preparing our consolidated financial statements could prove inaccurate.

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include those related to revenue recognition, accrued expenses and assumptions in the valuation of stock-based compensation. We base our estimates on historical experience, facts and circumstances known to us and on various other assumptions that we believe to be reasonable under the circumstances. We cannot provide assurances, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. If this is the case, we may

be required to restate our consolidated financial statements, which could, in turn, subject us to securities class action litigation. Defending against such potential litigation relating to a restatement of our consolidated financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation may be inadequate. As a result of these factors, any such potential litigation could have a material adverse effect on our financial results and cause our stock price to decline, which could in turn subject us to securities class action litigation.

Our ability to use net operating loss carryforwards and other tax attributes to offset future taxable income may be limited as a result of future transactions involving our common stock.

In general, under Section 382 of the Internal Revenue Code, a corporation that undergoes an ''ownership change'' is subject to limitations on its ability to utilize its pre-change net operating losses and certain other tax assets to offset future taxable income. In general, an ownership change occurs if the aggregate stock ownership of certain stockholders increases by more than 50 percentage points over such stockholders' lowest percentage ownership during the testing period, which is generally three years. An ownership change could limit our ability to utilize our net operating loss and tax credit carryforwards for taxable years including or following such "ownership change." Limitations imposed on the ability to use net operating losses and tax credits to offset future taxable income could require us to pay U.S. federal income taxes earlier than we estimated or than would have otherwise been required if such limitations were not in effect and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes.

If we fail to retain our key personnel or are unable to attract and retain additional qualified personnel, our future growth and our ability to compete would suffer.

We are highly dependent on the efforts and abilities of the principal members of our senior management. Additionally, we have scientific personnel with significant and unique expertise in RNA-targeted therapeutics and related technologies. The loss of the services of any one of the principal members of our managerial team or staff may prevent us from achieving our business objectives.

The competition for qualified personnel in the biotechnology field is intense, and our future success depends upon our ability to attract, retain and motivate such personnel. In order to develop and commercialize our products successfully, we will be required to retain key management and scientific employees. In certain instances, we may also need to expand or replace our workforce and our management ranks. In addition, we rely on certain consultants and advisors, including scientific and clinical advisors, to assist us in the formulation and advancement of our research and development programs. Our consultants and advisors may be employed by other entities or have commitments under consulting or advisory contracts with third parties that limit their availability to us, or both. If we are unable to attract, assimilate or retain such key personnel, our ability to advance our programs would be adversely affected.

If we are unable to effectively manage our growth, execute our business strategy and implement compliance controls and systems, the trading price of our common stock could decline. Any failure to establish and maintain effective internal control over financial reporting could adversely affect investor confidence in our reported financial information.

We anticipate continued growth in our business operations due, in part, to the commercialization of EXONDYS 51. This future growth could create a strain on our organizational, administrative and operational infrastructure. Our ability to manage our growth properly and maintain compliance with all applicable rules and regulations will require us to continue to improve our operational, legal, financial and management controls, as well as our reporting systems and procedures. We may not be able to build the management and human resources and infrastructure necessary to support the growth of our business. The time and resources required to implement systems and infrastructure that may be needed to support our growth is uncertain, and failure to complete implementation in a timely and efficient manner could adversely affect our operations.

We may engage in future acquisitions or collaborations with other entities that increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We actively evaluate various strategic transactions on an ongoing basis, including licensing or acquiring complementary products, technologies or businesses. Potential acquisitions or collaborations with other entities may entail numerous risks, including increased operating expenses and cash requirements, assimilation of operations and products, retention of key employees, diversion of our management's attention and uncertainties in our ability to maintain key business relationships of the acquired entities. In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

Our success, competitive position and future revenue, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our technologies and product candidates, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties.

We currently hold various issued patents and exclusive rights to issued patents and own and have licenses to various patent applications, in each case in the U.S. as well as other countries. We anticipate filing additional patent applications both in the U.S. and in other countries. The patent process, however, is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining and defending patents or in avoiding infringement of the rights of others. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect the technology owned by or licensed to us or our collaborators. Even if our patents and patent applications do provide our product candidates and platform technology with a basis for exclusivity, we and our collaborators may not be able to develop or commercialize such product candidates or platform technology due to patent positions held by one or more third parties.

We may not be able to obtain and maintain patent protection for our product or product candidates necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. For example, in July 2014, the Patent Trial and Appeal Board (the "PTAB") of the United States Patent and Trademark Office ("USPTO") declared patent interferences between certain patents held by Sarepta (under license from the University of Western Australia, "UWA") and patent applications held by BioMarin (under license from Academisch Ziekenhuis Leiden, "AZL") related to exon 51 and exon 53 skipping therapies designed to treat DMD. In particular, the PTAB declared Interference No. 106,008, which identifies Sarepta's/UWA's U.S. Patent Nos. 7,807,816 and 7,960,541, both covering EXONDYS 51, as interfering with BioMarin's/AZL's U.S. Application No. 13/550,210. The PTAB also declared Interference No. 106,007, which identifies Sarepta's/UWA's U.S. Patent No. 8,455,636, covering SRP-4053, as interfering with BioMarin's/AZL's U.S. Application No. 11/233,495. In September 2014, the PTAB declared a third patent interference relating to certain methods concerning the exon 51 skipping therapies that are the subject of Interference No. 106,008. In particular, the PTAB declared Interference No. 106,013, which identifies Sarepta's/UWA's U.S. Patent No. 8,486,907, which covers certain methods of using EXONDYS 51, as interfering with BioMarin's/AZL's U.S. Application No. 14/198,992. In addition, in a September 2014 Order in Interference No. 106,007, the PTAB authorized us to file a motion with the PTAB, which we filed in November 2014, requesting the declaration of a fourth interference relating to certain methods concerning the exon 53 skipping therapies that are the subject of Interference No. 106,007, including SRP-4053, and between Sarepta's/UWA's U.S. Patent No. 8,455,636 and BioMarin's/AZL's U.S. Application No. 14/248,279. In Interference No. 106,013, we received notice on September 29, 2015 that the PTAB had issued a decision that resulted in a judgment against Sarepta and an order for the cancellation of Sarepta's/UWA's U.S. Patent No. 8,486,907 that covers certain methods of using EXONDYS 51 thereby leaving open the possibility of BioMarin's/AZL's competing U.S. Application No. 14/198,992 to issue and, if so, potentially provide a basis for BioMarin to allege that EXONDYS 51 infringes a patent granting from this application. We filed a Request for Rehearing that requests the PTAB to continue this interference, and the PTAB denied our Request on December 29, 2015. We appealed this decision to the U.S. Court of Appeals for the Federal Circuit on March 28, 2016, and this appeal was docketed as Case Nos. 16-1937 (lead) & 16-2016 (consolidated). In Interference No. 106,007, the PTAB entered a judgment on the motions on April 29, 2016 to end this interference between U.S. Patent No. 8,455,636 held by Sarepta (under license from UWA) and U.S. Application No. 11/233,495 held by BioMarin (under license from AZL) related to exon 53 skipping therapies, including SRP-4053, designed to treat DMD. The PTAB ordered: (i) the final refusal of all claims of BioMarin's/AZL's U.S. Application No. 11/233,495, with the exception of claim 77; and (ii) cancellation of all claims in Sarepta's/UWA's U.S. Patent No. 8,455,636, in each case based on its decision of various motions. The PTAB denied our motion filed in November 2014 requesting the declaration of a fourth interference relating to certain methods concerning the exon 53 skipping therapies that are the subject of this Interference No. 106,007, including SRP-4053, and between Sarepta's U.S. Patent No. 8,455,636 and BioMarin's U.S. Application No. 14/248,279, thereby leaving open the possibility of BioMarin's/AZL's competing U.S. Application No. 14/248,279 to issue and, if so, potentially provide a basis for BioMarin to allege that our product candidate, SRP-4053, infringes a patent granting from this application. BioMarin appealed the decision from Interference No. 106,007 to the U.S. Court of Appeals for the Federal Circuit on June 28, 2016, and this appeal was docketed as Case No. 16-2262 and designated by the Court as a companion case to the exon 51 methods interference appeal (Case No. 16-1937). On September 20, 2016, the PTAB issued a judgment in Interference No. 106,008 against BioMarin/AZL and ordered the final result of all claims of AZL's application, U.S. Application No. 13/550,210. BioMarin/AZL may request rehearing before the PTAB and/or appeal the decision to the U.S. Court of Appeals for the Federal Circuit. We cannot make any assurances about the outcome of any rehearing decisions or appeals of any of these three interferences. Any adverse rulings on rehearing or appeal could come at any time and, if negative, could adversely affect our business and result in a decline in our stock price. If final resolution of the interference and related appeals are not in our favor, then the Sarepta/UWA patents involved in the interference, any other Sarepta/UWA patents or applications also found to be interfering, and any other Sarepta/UWA patents or applications may be invalidated or subject to invalidation, and as a result, we may not have any patent-based exclusivity available for our product or product candidates, which may have a material negative impact on our business plans. In addition, if final resolution of the interference or related appeals are not in our favor, the USPTO may issue the BioMarin/AZL patent applications resulting in the grant of one or more patents that may provide a basis for BioMarin to allege that EXONDYS 51 and/or our product candidate, SRP-4053, infringe such patents. In addition, the interference, appeals and any

subsequent litigation may require significant financial resources that we may have planned to spend on other Company objectives, resulting in delays or other negative impacts on such other objectives. In addition, BioMarin may continue to evaluate other opportunities to challenge our intellectual property rights or seek to broaden their patent positions in an attempt to cover our product candidates in the U.S. and in other jurisdictions. We are also aware of certain pending and granted claims that are held by BioMarin in Japan, Europe and certain other countries that may provide the basis for BioMarin or other parties to assert that EXONDYS 51 infringes on such claims. Because we have not yet initiated an invalidation proceeding in these countries, the outcome and timing of any such proceeding cannot be predicted or determined as of the date of this report.

As a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection or impose compulsory licenses for disease treatments that prove successful. Additionally, jurisdictions other than the U.S. might have less restrictive patent laws than the U.S., giving foreign competitors the ability to exploit these laws to create, develop and market competing products. The USPTO and patent offices in other jurisdictions have often required that patent applications concerning pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Accordingly, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated.

On September 16, 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act"), was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted, and may also affect patent litigation. The USPTO has issued regulations and procedures to govern administration of the Leahy-Smith Act, but many of the substantive changes to patent law associated with the Leahy-Smith Act have only recently become effective. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition. For instance, a third party may petition the PTAB seeking to challenge the validity of some or all of the claims in any of our patents through an *Inter Partes Review* ("IPR") or other post-grant proceeding. Should the PTAB institute an IPR (or other) proceeding and decide that some or all of the claims in the challenged patent are invalid, such a decision, if upheld on appeal, could have a material adverse effect on our business and financial condition.

The full impact of several recent U.S. Supreme Court decisions relating to patent law is not yet known. For example, on March 20, 2012, in *Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc* ., the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. The decision appears to impact diagnostics patents that merely apply a law of nature via a series of routine steps and it has created uncertainty around the ability to patent certain biomarker-related method claims. Additionally, on June 13, 2013, in *Association for Molecular Pathology v. Myriad Genetics, Inc.* , the Court held that claims to isolated genomic DNA are not patentable, but claims to complementary DNA molecules were held to be valid. The effect of the decision on patents for other isolated natural products is uncertain and, as with the Leahy-Smith Act, these decisions could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Our business prospects will be impaired if third parties successfully assert that EXONDYS 51 or our product candidates or technologies infringe proprietary rights of such third parties.

Our competitors may make significant investments in competing technologies, and might have or obtain patents that limit, interfere with or eliminate our ability to make, use and sell EXONDYS 51 or our product candidates in important commercial markets.

If EXONDYS 51 or our product candidates or technologies infringe enforceable proprietary rights of others, we could incur substantial costs and may have to:

- obtain rights or licenses from others, which might not be available on commercially reasonable terms or at all;
- abandon development of an infringing product candidate;
- redesign EXONDYS 51, product candidates or processes to avoid infringement;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could substantially harm our potential earnings, financial condition and operations. BioMarin has rights to patent claims that, absent a license, may preclude us from commercializing EXONDYS 51 in several jurisdictions. BioMarin has

rights to European Patent No. EP 1619249, for example. We opposed this patent in the Opposition Division of the European Patent Office ("EPO"), and the Opposition Division maintained certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46, which may provide a basis to maintain that commercialization of EXONDYS 51 in a European country where BioMarin has a patent corresponding to EP 1619249 would infringe on such patent. Both we and BioMarin have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other's briefs. BioMarin filed arguments with the EPO in response to Sarepta's previously filed briefs. The Opposition Division decision, if maintained at the appeals level, could have a substantial negative effect on our business and leaves open the possibility that BioMarin or other parties that have rights to such patent could assert that EXONDYS 51 infringes on such patent in a relevant European country. The timing and outcome of the appeal cannot be predicted or determined as of the date of this report. If as part of any appeal before the EPO we are unsuccessful in invalidating BioMarin's claims that were maintained by the Opposition Division or if claims previously invalidated by the Opposition Division are restored on appeal, our ability to commercialize both EXONDYS 51 and our therapeutic candidates could be materially impaired. Moreover, our ability to commercialize EXONDYS 51 in a European country where BioMarin has a patent related to EP 1619249 while the appeal process remains ongoing before the EPO Board of Appeals could be materially impaired. In addition, we are aware of various divisional applications relating to EP 1619249 that are being pursued by BioMarin, which are pending and in some cases are granted. Any of these granted patents can also materially impair our ability to commercialize EXONDYS 51 or our therapeutic candidates, such as SRP-4045 and SRP-4053.

We are also aware of existing patent claims BioMarin is pursuing in the U.S., including those involved in the interferences declared by the USPTO in July 2014 and September 2014 and discussed in these risk factors, and others that it has or is pursuing in other countries, that where granted may provide the basis for BioMarin or other parties to assert that commercialization of EXONDYS 51 and certain other of our product candidates would infringe on such claims. Some of these existing patent claims have granted and may provide a basis for BioMarin to allege that EXONDYS 51 infringes such granted claims. These patent claims may materially impair our ability to commercialize EXONDYS 51.

The DMD patent landscape is continually evolving and multiple parties, including both commercial entities and academic institutions, may have rights to claims or may be pursuing additional claims that could provide these parties a basis to assert that EXONDYS 51 or our product candidates infringe on the intellectual property rights of such parties. Similarly, we may be able to assert that certain activities engaged in by these parties infringe on our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. We also cannot be certain that other third parties will not assert patent infringement in the future with respect to any of our development programs.

We face intense competition and rapid technological change, which may result in other companies discovering, developing or commercializing competitive products.

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change. We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in areas related to antisense technology and other RNA technologies, or that are developing alternative approaches to or therapeutics for the disease indications on which we are focused. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with EXONDYS 51 or our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals, Inc.), Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Wave Life Sciences ("Wave"), and Nippon Shinyaku Co. Ltd. share a focus on RNA-targeted drug discovery and development. Competitors with respect to EXONDYS 51 include BioMarin (which acquired Prosensa), Nippon Shinyaku, Daiichi Sankyo, Wave and Shire plc; and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs. Additionally, several companies have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Pfizer, Inc., Bristol-Myers Squibb, Biogen Idec, Inc., Ionis Pharmaceuticals, Inc., Alexion Pharmaceuticals, Inc., Sanofi, Eli Lilly, Alnylam, Moderna Therapeutics, Inc., Summit plc, Akashi, Catabasis, and Oxford University. Although BioMarin received a complete response letter for Kyndrisa ™ (drisapersen) for the treatment of DMD in patients with mutations that are amenable to exon 51 skipping on January 14, 2016, BioMarin continues to be a competitor for us on the development of DMD exon-skipping product candidates.

On May 31, 2016, BioMarin announced the withdrawal of its market Authorization Application for Kyndrisa™ (drisapersen) in Europe and its intent to discontinue clinical and regulatory development of Kyndrisa ™ and three other follow-on products, BMN 044, BMN 045 and BMN 053. If BioMarin or any of our competitors are successful in obtaining regulatory approval for any of their product candidates, it may limit our ability to gain or keep market share in the DMD space or other diseases targeted by our exon-skipping platform and product candidate pipeline.

It is possible that our competitors will succeed in developing technologies that limit the market size for EXONDYS 51 or our product candidates, impact the regulatory approval process for our product candidates that are more effective than our product candidates or that would render our technology obsolete or noncompetitive. Our competitors, including BioMarin, may, among other things:

- develop safer or more effective products;
- implement more effective approaches to sales and marketing;
- develop less costly products;
- obtain regulatory approval more quickly;
- have access to more manufacturing capacity;
- develop products that are more convenient and easier to administer;
- form more advantageous strategic alliances; or
- establish superior intellectual property positions.

We may be subject to product liability claims and our insurance may not be adequate to cover damages.

The current and future use of our product candidates by us and our collaborators in clinical trials, expanded access programs, the sale of EXONDYS 51 and future products, or the use of our products under emergency use vehicles may expose us to liability claims inherent to the manufacture, clinical testing, marketing and sale of medical products. These claims might be made directly by consumers or healthcare providers or indirectly by pharmaceutical companies, our collaborators or others selling such products. Regardless of merit or eventual outcome, we may experience financial losses in the future due to such product liability claims. We have obtained limited general commercial liability insurance coverage for our clinical trials. We intend to expand our insurance coverage to include the sale of commercial products in connection with the FDA's approval of EXONDYS 51. However, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against all losses. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

Our operations involve the use of hazardous materials, and we must comply with environmental laws, which can be expensive, and may affect our business and operating results.

Our research and development activities involve the use of hazardous materials, including organic and inorganic solvents and reagents. Accordingly, we are subject to federal, state and local laws and regulations governing the use, storage, handling, manufacturing, exposure to and disposal of these hazardous materials. In addition, we are subject to environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. Although we believe that our activities conform in all material respects with such environmental laws, there can be no assurance that violations of these laws will not occur in the future as a result of human error, accident, equipment failure or other causes. Liability under environmental, health and safety laws can be joint and several and without regard to fault or negligence. The failure to comply with past, present or future laws could result in the imposition of substantial fines and penalties, remediation costs, property damage and personal injury claims, loss of permits or a cessation of operations, and any of these events could harm our business and financial condition. We expect that our operations will be affected by other new environmental, health and workplace safety laws on an ongoing basis, and although we cannot predict the ultimate impact of any such new laws, they may impose greater compliance costs or result in increased risks or penalties, which could harm our business.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cyber security incidents, could harm our ability to operate our business effectively.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, our proprietary business information and that of our suppliers, as well as personally identifiable information of EXONDYS 51 patients, clinical trial participants and employees. Similarly, our third-party providers possess certain of our sensitive data. The secure maintenance of this information is critical to our operations and business strategy. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information, including our data being breached at third-party providers, could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations and damage our reputation, which could adversely affect our business.

We may incur substantial costs in connection with litigation and other disputes.

In the ordinary course of business we may, and in some cases have, become involved in lawsuits and other disputes such as securities claims, intellectual property challenges, including interferences declared by the USPTO, and employee matters. It is possible that we may not prevail in claims made against us in such disputes even after expending significant amounts of money and company resources in defending our positions in such lawsuits and disputes. The outcome of such lawsuits and disputes is inherently uncertain and may have a negative impact on our business, financial condition and results of operations.

Risks Related to Our Common Stock

Our stock price is volatile and may fluctuate due to factors beyond our control.

The market prices for and trading volumes of securities of biotechnology companies, including our securities, has historically been volatile. Our stock has had significant swings in trading prices, in particular in connection with our public communications regarding feedback received from regulatory authorities. For example, over the last twelve months, our stock has increased as much as 74% in a single day or decreased as much as 55% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

- the commercial performance of EXONDYS 51 in the U.S.;
- the timing of our submissions to regulatory authorities and regulatory decisions and developments;
- positive or negative clinical trial results or regulatory interpretations of data collected in clinical trials conducted by us, our strategic partners, our competitors or other companies with investigational drugs targeting the same, similar or related diseases to those targeted by us;
- delays in beginning and completing pre-clinical and clinical studies for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of EXONDYS 51 or our product candidates or entry into strategic relationships on terms that are not deemed to be favorable to our Company;
- technological innovations, product development or additional commercial product introductions by ourselves or competitors;
- changes in applicable government regulations or regulatory requirements in the approval process;
- developments concerning proprietary rights, including patents and patent litigation matters, such as developments in the interferences declared by the USPTO, including in the near term any outcomes of ongoing interference proceedings and over the longer term the outcomes from any related appeals;
- public concern relating to the commercial value, efficacy or safety of any of our products;
- our ability to obtain funds, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;
- comments by securities analysts;
- developments in litigation such as the stockholder lawsuits against us;
- changes in senior management; or
- general market conditions in our industry or in the economy as a whole.

Broad market and industry factors may seriously affect the market price of a company's stock, including ours, regardless of actual operating performance. In addition, in the past, following periods of volatility in the overall market and the market price of a particular company's securities, securities class action litigation has often been instituted against these companies. Such litigation could result in substantial costs and a diversion of our management's attention and resources.

Provisions of our certificate of incorporation, bylaws and Delaware law might deter acquisition bids for us that might be considered favorable and prevent or frustrate any attempt to replace or remove the then-current management and board of directors.

Certain provisions of our certificate of incorporation and bylaws may make it more difficult for a third party to acquire control of us or effect a change in our board of directors and management. These provisions include:

- when the board is comprised of six or more directors, classification of our board of directors into two classes, with one class elected each year;
- directors may only be removed for cause by the affirmative vote of a majority of the voting power of all the then-outstanding shares of voting stock;
- prohibition of cumulative voting of shares in the election of directors;
- right of the board of directors to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death, disqualification or removal of a director;
- express authorization of the board of directors to make, alter or repeal our bylaws;
- prohibition on stockholder action by written consent;
- advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at stockholder meetings;
- the ability of our board of directors to authorize the issuance of undesignated preferred stock, the terms and rights of which may be established and shares of which may be issued without stockholder approval, including rights superior to the rights of the holders of common stock; and
- a super-majority (66 2/3%) of the voting power of all of the then-outstanding shares of capital stock are required to amend, rescind, alter or repeal our bylaws and certain provisions of our certificate of incorporation.

In addition, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These and other provisions in our certificate of incorporation and our bylaws and in the Delaware General Corporation Law could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors.

We expect our operating results to fluctuate in future periods, which may adversely affect our stock price.

Our quarterly operating results have fluctuated in the past, and we believe they will continue to do so in the future. Our operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Likewise, our research and development expenses may experience fluctuations as a result of the timing and magnitude of expenditures incurred in support of our proprietary drug development programs. In one or more future periods, our results of operations may fall below the expectations of securities analysts and investors. In that event, the market price of our common stock could decline.

A significant number of shares of our common stock are issuable pursuant to outstanding stock awards, and we expect to issue additional stock awards and shares of common stock in the future. Exercise of these awards and sales of shares will dilute the interests of existing security holders and may depress the price of our common stock.

As of September 30, 2016, there were approximately 54.4 million shares of common stock outstanding and outstanding awards to purchase 6.3 million shares of common stock under various incentive stock plans. Additionally, as of September 30, 2016, there were approximately 2.8 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, approximately 0.3 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and approximately 1.1 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our Amended and Restated 2011 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. The issuance of additional shares of common stock or warrants to purchase common stock and the perception that such issuances may occur or exercise of outstanding warrants or options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

The exhibits listed on the Exhibit Index immediately preceding such exhibits, which is incorporated herein by reference, are filed or furnished as part of this Quarterly Report on Form 10-Q.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

SAREPTA THERAPEUTICS, INC.
(Registrant)

Date: November 7, 2016

By: /s/ EDWARD KAYE, MD
Edward Kaye, MD
President, Chief Executive Officer and Chief Medical Officer
(Principal Executive Officer)

Date: November 7, 2016

By: /s/ SANDESH MAHATME
Sandesh Mahatme
Senior Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
4.1	Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan	8-K	001-14895	10.1	07/01/2016	
4.2	Sarepta Therapeutics, Inc. Amended and Restated 2013 Employee Stock Purchase Plan	8-K	001-14895	10.2	07/01/2016	
10.1	Executive Employment Agreement dated September 20, 2016 by and between Sarepta Therapeutics, Inc. and Edward M. Kaye, M.D.					X
10.2	License and Collaboration Agreement by and between Summit (Oxford) Ltd. and Sarepta Therapeutics, Inc. dated October 3, 2016.					X
31.1	Certification of the Company's Interim Chief Executive Officer, Edward Kaye, MD, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of the Company's Interim Chief Executive Officer, Edward Kaye, MD, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

* The Certifications attached as Exhibits 32.1 and 32.2 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the SEC and are not to be incorporated by reference into any filings of Sarepta Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

Exhibit 10.1

Sarepta Therapeutics, Inc.

EMPLOYMENT AGREEMENT

EMPLOYMENT AGREEMENT (this "Agreement") dated as of September 20, 2016, between **Sarepta Therapeutics, Inc.**, a Delaware corporation (the "Company"), and **Edward M. Kaye, M.D.** (the "Executive").

W I T N E S S E T H

WHEREAS, the Company desires to employ the Executive as the President and Chief Executive Officer of the Company; and

WHEREAS, the Company and the Executive desire to enter into this Agreement as to the terms of the Executive's employment with the Company.

NOW, THEREFORE, in consideration of the foregoing, of the mutual promises contained herein and of other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto hereby agree as follows:

1.POSITION AND DUTIES.

(a)During the Employment Term (as defined in Section 2 hereof), the Executive shall serve as the President and Chief Executive Officer of the Company. In this capacity, the Executive shall have the duties, authorities and responsibilities commensurate with the duties, authorities and responsibilities of persons in similar capacities in similarly sized companies, and such other duties, authorities and responsibilities as the Board of Directors of the Company (the "Board") shall designate from time to time that are not inconsistent with the Executive's position as the President and Chief Executive Officer of the Company. The Executive shall report to the Board.

(b)During the Employment Term, the Executive shall devote all of the Executive's business time, energy and skill and the Executive's efforts to the performance of the Executive's duties with the Company, provided that the foregoing shall not prevent the Executive from (i) serving on up to one board of directors of a non-profit organization and/or, with the prior written approval of the Board, one for-profit company board of directors, (ii) participating in charitable, civic, educational, professional, community or industry affairs, and (iii) managing the Executive's passive personal investments so long as such activities, individually or in the aggregate, do not materially interfere or conflict with the Executive's duties hereunder or create a potential business or fiduciary conflict.

(c)The Executive shall become a member of the Board as of the Effective Date (as defined in Section 2 hereof). Subject to Section 8(e), during the Employment Term the Board shall nominate the Executive for re-election as a member of the Board at the expiration of the then current term, provided that the foregoing shall not be required to the extent prohibited by legal or regulatory requirements.

2.EMPLOYMENT TERM. The Company agrees to employ the Executive under and pursuant to the terms of this Agreement, and the Executive agrees to be so employed as President and Chief Executive Officer, for an initial term of one year (the "Initial Term") commencing as of the date hereof (the "Effective Date"). At the conclusion of the Initial Term, and on each anniversary of the Effective Date following the Initial Term, the term of this Agreement shall be automatically extended for successive one-year periods, provided, however, that either party hereto may elect not to extend the term of this Agreement by giving written notice to the other party at least 60 days prior to any such date. Notwithstanding the foregoing, the Executive's employment hereunder may be terminated prior to the end of the then current Employment Term (as defined below) in accordance with Section 7 hereof, subject to Section 8 hereof. The period of time between the Effective Date and the termination or expiration of the term of this Agreement shall be referred to herein as the "Employment Term." Except as otherwise agreed in writing by the Company and the Executive, this Agreement shall terminate (without renewal or extension) upon the expiration of the Employment Term. Employment thereafter, if any, shall be on an on an at-will basis. For the avoidance of doubt, a non-renewal of the Agreement by the Company shall not constitute a termination of the executive's employment by the Company without Cause and a non-renewal of the Agreement by the Executive shall not constitute a termination of the Executive's employment by Executive for Good Reason, and if Executive's employment with the Company terminates at such time of non-renewal (other than for Cause), the Executive shall be entitled to receive the Accrued Benefits (as defined in Section 8(a)), and such Accrued Benefits shall be the only amount that Executive is entitled to receive pursuant to this Agreement. For the avoidance of doubt, the termination or expiration of this Agreement shall not operate to terminate that certain Change in Control Agreement entered into between Executive and the Company as of November 7, 2013 (the "Severance Agreement").

3.BASE SALARY. The Company agrees to pay the Executive a base salary at an annual rate of not less than $550,000, payable in accordance with the regular payroll practices of the Company, but not less frequently than monthly. The Executive's Base Salary shall be subject to annual review by the Board (or a committee thereof), and may be increased, but not decreased below its then current level, from time to time by the Board. The base salary as determined herein from time to time shall constitute "Base Salary" for purposes of this Agreement.

4.ANNUAL BONUS. During the Employment Term, the Executive shall be eligible to receive an annual discretionary incentive payment under the Company's annual bonus plan as in effect from time to time (the "Annual Bonus") based on a target bonus opportunity of at least 65% of the Executive's Base Salary (the "Target Bonus"), upon the attainment of one or more pre-established performance goals established by the Board or the Company's Compensation Committee (the "Committee") following good-faith consultation with the Executive. To the extent determined by the Committee, all or any portion of Executive's Annual Bonus may be paid in the form of equity compensation awards under the Company's Amended and Restated 2011 Equity Incentive Plan, as amended and/or restated from time to time, or any successor shareholder-approved Company equity compensation plan. Any portion of the Annual Bonus payable in cash shall be deemed "earned" if the Executive is employed on the last day of the applicable year, and the Annual Bonus, whether paid in cash or equity, shall be paid or delivered no later than March 15th of the calendar year immediately following the applicable year to which the Annual Bonus relates.

5.EQUITY AWARDS. Beginning in 2016 and in each calendar year during the Employment Term thereafter, the Executive shall be eligible to receive a long-term incentive award commensurate with the Executive's position as the President Chief Executive Officer of the Company, in such amount and form, and subject to such terms and conditions, as may be determined by the Board or the Committee.

6.EMPLOYEE BENEFITS.

(a)**BENEFIT PLANS.** The Executive shall be entitled to participate in any employee benefit plan that the Company has adopted or may adopt, maintain or contribute to for the benefit of its employees generally, on the same basis as those benefits are generally made available to other executives of the Company, subject to satisfying the applicable eligibility requirements.

(b)**VACATIONS.** The Executive shall be entitled to paid vacation in accordance with the Company's policy on accrual and use applicable to employees as in effect from time to time.

(c)**BUSINESS AND TRAVEL EXPENSES.** Upon presentation of appropriate documentation, the Executive shall be reimbursed in accordance with the Company's expense reimbursement policy, for all reasonable business expenses incurred in connection with the performance of the Executive's duties hereunder and the Company's policies with regard thereto.

7.TERMINATION. The Executive's employment and the Employment Term hereunder shall terminate on the first of the following to occur:

(a)**DISABILITY.** Upon thirty (30) days' prior written notice by the Company to the Executive of termination due to Disability. For purposes of this Agreement, "Disability" shall be defined as the inability of the Executive to have performed the Executive's material duties hereunder due to a physical or mental injury, infirmity or incapacity for one hundred eighty (180) days (including weekends and holidays) in any 365-day period. Notwithstanding the foregoing, in the event that as a result of earlier absence because of mental or physical incapacity Executive incurs a "separation from service" within the meaning of such term under "Code Section 409A" (as defined in Section 25(a) hereof), Executive shall on such date automatically be terminated from employment as a Disability termination.

(b)**DEATH.** Automatically on the date of death of the Executive.

(c)**CAUSE.** Subject to this Section 7(c), immediately upon written notice by the Company to the Executive of a termination for Cause. "Cause" shall mean:

(i)The Executive's substantial and repeated failure to attempt in good faith to perform the Executive's duties or follow the reasonable and legal written direction of the Board;

(ii)The Executive's willful material misconduct with respect to any material aspect of the business of the Company;

(iii)The indictment for, conviction of, or pleading of guilty or nolo contendere to, a felony or any crime involving moral turpitude;

(iv) The Executive's performance of any material act of theft, fraud or malfeasance in connection with the performance of the Executive's duties to the Company; or

(v) A material breach of this Agreement or a material violation of the Company's code of conduct or other written material policy.

(d) **WITHOUT CAUSE.** Immediately upon written notice by the Company to the Executive of an involuntary termination without Cause (other than for death or Disability).

(e) **GOOD REASON.** Upon thirty (30) days' prior written notice by the Executive to the Company of a termination for Good Reason. "Good Reason" shall mean the occurrence of any of the following events:

(i) Material diminution in the Executive's Base Salary or Target Bonus;

(ii) Material diminution in the Executive's title or responsibilities (other than temporarily while physically or mentally incapacitated or as required by applicable law);

(iii) Relocation of the Executive's primary work location by more than 50 miles from its then current location; or

(iv) A material breach by the Company of this Agreement or any equity award agreement, including, without limitation, the removal of the Executive from the Board by the Company (other than for Cause) or the failure to nominate the Executive for re-election to serve on the Board

The Executive shall provide the Company with a written notice detailing the specific circumstances alleged to constitute Good Reason within ninety (90) days after the first occurrence of such circumstances, and the Company shall have thirty (30) days following receipt of such notice to cure such circumstances in all material respects, provided, that, no termination for Good Reason shall occur after the 180th day following the first occurrence of any Good Reason event.

(f) **WITHOUT GOOD REASON.** Upon thirty (30) days' prior written notice by the Executive to the Company of the Executive's voluntary termination of employment without Good Reason (which the Company may, in its sole discretion, make effective earlier than any notice date).

8. CONSEQUENCES OF TERMINATION.

(a) **DEATH OR DISABILITY.** In the event that the Executive's employment is terminated due to the Executive's death or Disability, the Executive or the Executive's legal representative or estate, as the case may be, shall be entitled to the following:

(i) The "Accrued Benefits," which shall mean: (A) any earned but unpaid Base Salary through the date of termination, payable in accordance with the regular payroll practices of the Company, but no later than thirty (30) days following the date of termination; (B) any Annual Bonus earned but unpaid with respect to the fiscal year ending

on or preceding the date of termination, payable at the time such bonuses would have been paid if the Executive was still employed with the Company; (C) reimbursement for any unreimbursed business expenses incurred through the date of termination within thirty (30) days following the date of termination; (D) any accrued but unused vacation time in accordance with Company policy; and (E) all other payments, benefits or fringe benefits to which the Executive shall be entitled under the terms of any applicable compensation arrangement or benefit, equity or fringe benefit plan or program or grant or this Agreement; and

(ii) Any additional vesting of any equity awards pursuant to and in accordance with the terms of the 2011 Sarepta Therapeutics Equity Incentive Plan ("Plan"), as amended, and equity award agreements ("Equity Agreements") executed in connection with each equity grant by the Company to Executive.

Notwithstanding anything to the contrary in this Section 8(a), in the event of a termination for disability, Executive shall be paid any earned but unpaid Base Salary and accrued but unused vacation time on the date of termination.

(b) **TERMINATION FOR CAUSE.** If the Executive's employment is terminated by the Company for Cause, the Company shall pay to the Executive the Accrued Benefits (other than the benefit described in Section 8(a)(i)(B) hereof). Notwithstanding anything to the contrary in Section 8(a), in the event of a termination for cause, Executive shall be paid any earned but unpaid Base Salary and accrued but unused vacation time on the date of termination.

(c) **TERMINATION WITHOUT GOOD REASON OR AS A RESULT OF NON-EXTENSION OF THIS EMPLOYMENT TERM**. If the Executive's employment is terminated by (x) the Executive without Good Reason or (y) as a result of non-extension of the Employment Term as provided in Section 2 hereof, the Company shall pay to the Executive the Accrued Benefits.

(d) **TERMINATION WITHOUT CAUSE OR FOR GOOD REASON OUTSIDE OF CHANGE IN CONTROL PERIOD.** If during the Employment Term and outside of a Change in Control Period (as defined in the Severance Agreement), the Executive's employment by the Company is terminated (x) by the Company without Cause, or (y) by the Executive for Good Reason, the Company shall pay or provide the Executive with the following, subject to the provisions of Section 22 hereof:

(i) The Accrued Benefits;

(ii) The Pro-Rata Bonus;

(iii) An aggregate amount equal to the sum of (1) 18 months of the Executive's then current Base Salary and (2) the Executive's Target Bonus, payable in substantially equal instalments in accordance with the regular payroll policies of the Company, over a period of 18 months following the date of termination of employment, except that the first installment shall be paid on the sixtieth (60^{th}) day following the date of termination and shall include any prior installment that would have been payable if the Release requirement set forth in Section 9 were satisfied on the date of termination;

(iv) A monthly amount equal to the monthly amount of the COBRA continuation coverage premium under the Company's group medical plans as in effect from time to time less the amount of the Executive's portion of the premium as if Executive was an active employee for a period of 18 months, except that the first installment shall be paid on the sixtieth (60th) day following the date of termination and shall include any prior installment that would have been payable if the Release requirement set forth in Section 9 were satisfied on the date of termination;

(v) Each outstanding unvested equity award issued to Executive as of the date of termination and scheduled to vest over the 12 months following the date of termination, (excluding, for this purpose, any performance-based grants ("Performance Awards") which are addressed below) shall automatically become vested and, if applicable, exercisable and any forfeiture restrictions or rights of repurchase thereto shall immediately lapse. For Performance Awards, each outstanding unvested Performance Award eligible for vesting as a result of having achieved performance milestones prior to the date of termination and scheduled to vest over the 12 months following the date of termination shall automatically become vested and, if applicable, exercisable and any forfeiture restrictions or rights of repurchase thereto shall immediately lapse. For the sake of clarity, to the extent a performance milestone under a Performance Award is not met as of the termination date, no award grants or vesting contingent on the achievement of such milestone or milestones shall be eligible for accelerated vesting even if met at a later date. Notwithstanding the foregoing, the Compensation Committee may at its sole discretion authorize the accelerated vesting of a portion of or all unvested Performance Awards to the extent permitted under the plan, but shall be under no obligation to do so. Any remaining unvested equity awards as of the termination date shall be immediately forfeited and of no further force or effect.

(vi) Outplacement services at a level commensurate with the Executive's position in accordance with the Company's practices as in effect from time to time *provided* that the cost of such outplacement shall not exceed $20,000; and *provided, further,* that such outplacement benefits shall end not later than the last day of the second calendar year that begins after the date of termination.

(e) **OTHER OBLIGATIONS.** Upon any termination of the Executive's employment with the Company, the Executive shall promptly resign from the Board and any other position as an officer, director or fiduciary of any Company-related entity. To the extent Executive remains an employee of the Company but, is no longer serving in the capacity of the Chief Executive Officer, Executive shall promptly resign from the Board upon such change in responsibilities.

(f) **EXERCISE PERIOD.** In the event of a termination without Cause by the Company or termination for Good Reason by Executive, Executive shall have no less than 12 months from the date of termination (but in no event beyond the remaining term of such equity awards) to exercise any equity awards (a) already vested as of the date of termination or (b) vested in accordance with Section 8(d)(v). In the event of termination for any reason other than without cause or Good Reason, the time period to exercise any equity awards already vested as of the date of termination shall be as set forth in the Plan or Equity Agreements.

(g)**TERMINATION DURING A CHANGE IN CONTROL PERIOD.** In the event of a termination of the Executive's employment with the Company during the Change in Control Period that is coincident with the Employment Term, then Executive shall be entitled to severance benefits under Severance Agreement in accordance with its terms and conditions.

9. RELEASE; NO MITIGATION. Any and all amounts payable and benefits or additional rights provided pursuant to Section 8(d) hereof beyond the Accrued Benefits shall only be payable only if the Executive delivers to the Company and does not revoke a general release of claims in favor of the Company substantially in the same form as attached hereto as Exhibit A (the "Release"). Such Release shall be executed and delivered (and no longer subject to revocation, if applicable) within sixty (60) days following the date of termination; provided that the Company delivers to Executive such Release within seven (7) days after the date of termination. In no event shall the Executive be obligated to seek other employment or take any other action by way of mitigation of the amounts payable to the Executive under any of the provisions of this Agreement, nor shall the amount of any payment hereunder be reduced by any compensation earned by the Executive as a result of employment by a subsequent employer, except as provided in Section 8(d)(iv) hereof.

10.RESTRICTIVE COVENANTS.

(a)**CONFIDENTIALITY.** The Executive agrees that the Executive shall not, directly or indirectly, use, make available, sell, disclose or otherwise communicate to any person, other than in the course of the Executive's assigned duties and for the benefit of the Company, either during the period of the Executive's employment or at any time thereafter, any business and technical information or trade secrets, nonpublic, proprietary or confidential information, knowledge or data relating to the Company, any of its subsidiaries, affiliated companies or businesses, which shall have been obtained by the Executive during the Executive's employment by the Company (or any predecessor). The foregoing shall not apply to information that (i) was known to the public prior to its disclosure to the Executive; (ii) becomes generally known to the public subsequent to disclosure to the Executive through no wrongful act of the Executive or any representative of the Executive; or (iii) the Executive is required to disclose by applicable law, regulation or legal process (provided that the Executive provides the Company with prior notice of the contemplated disclosure and cooperates with the Company at its expense in seeking a protective order or other appropriate protection of such information). In addition, nothing in this Agreement shall be construed to prohibit the Executive from reporting possible violations of federal or state law or regulations to any governmental agency or self-regulatory organization with oversight responsibility for the Company, or making other disclosures that are protected under whistleblower or other provisions of any applicable federal or state law or regulations. Prior authorization of the Company is not required to make any such reports or disclosures, and the Executive is not required to notify the Company that he has made such reports or disclosures.

(b)**NONCOMPETITION.** The Executive acknowledges that the Executive performs services of a unique nature for the Company that are irreplaceable, and that the Executive's performance of such services to a competing business will result in irreparable harm to the Company. Accordingly, during the Executive's employment hereunder and for a period of one (1) year thereafter, the Executive agrees that the Executive will not, directly or indirectly, own, manage, operate, control, be employed by (whether as an employee, consultant, independent

contractor or otherwise, and whether or not for compensation) or render services to any person, firm, corporation or other entity, in whatever form, engaged in the research, development or sale of Duchenne Muscular Dystrophy treatments, oligonucleotide based therapies, or chemistry platforms that compete with Company or any of its subsidiaries or affiliates or in any other material business in which the Company or any of its subsidiaries or affiliates is engaged on the date of termination or in which they have planned, on or prior to such date, to be engaged in on or after such date, in any locale of any country in which the Company conducts business. Notwithstanding the foregoing, nothing herein shall prohibit the Executive from being a passive owner of not more than one percent (1%) of the equity securities of a publicly traded corporation engaged in a business that is in competition with the Company or any of its subsidiaries or affiliates, so long as the Executive has no active participation in the business of such corporation. In addition, the provisions of this Section 10(b) shall not be violated by the Executive commencing employment with a subsidiary, division or unit of any entity that engages in a business in competition with the Company or any of its subsidiaries or affiliates so long as the Executive and such subsidiary, division or unit does not engage in a business in competition with the Company or any of its subsidiaries or affiliates.

(c)**NONSOLICITATION; NONINTERFERENCE; NONDISPARAGEMENT.** (i) During the Executive's employment with the Company and for a period of one (1) year thereafter, the Executive agrees that the Executive shall not, except in the furtherance of the Executive's duties hereunder, directly or indirectly, individually or on behalf of any other person, firm, corporation or other entity, solicit, aid or induce any customer of the Company or any of its subsidiaries or affiliates to purchase goods or services then sold by the Company or any of its subsidiaries or affiliates from another person, firm, corporation or other entity or assist or aid any other persons or entity in identifying or soliciting any such customer.

(ii)During the Executive's employment with the Company and for a period of one (1) year thereafter, the Executive agrees that the Executive shall not, except in the furtherance of the Executive's duties hereunder, directly or indirectly, individually or on behalf of any other person, firm, corporation or other entity, (A) solicit, aid or induce any employee, representative or agent of the Company or any of its subsidiaries or affiliates to leave such employment or retention or to accept employment with or render services to or with any other person, firm, corporation or other entity unaffiliated with the Company or hire or retain any such employee, representative or agent, or take any action to materially assist or aid any other person, firm, corporation or other entity in identifying, hiring or soliciting any such employee, representative or agent, or (B) interfere, or aid or induce any other person or entity in interfering, with the relationship between the Company or any of its subsidiaries or affiliates and any of their respective vendors, joint venturers or licensors. An employee, representative or agent shall be deemed covered by this Section 10(c)(ii) while so employed or retained and for a period of six (6) months thereafter, or (C) either publicly or privately, disparage, criticize or defame the Company, its affiliates and their respective affiliates, directors, officers, agents, partners, stockholders, individuals or the Company's, products, services, technology or business.

(iii)Notwithstanding the foregoing, the provisions of this Section 10(c) shall not be violated by (A) general advertising or solicitation not specifically targeted at Company-related persons or entities, (B) the Executive serving as a reference, upon request, for any

employee of the Company or any of its subsidiaries or affiliates, or (C) actions taken by any person or entity with which the Executive is associated if the Executive is not personally involved in any manner in the matter and has not identified such Company-related person or entity for soliciting or hiring.

(d)**RETURN OF COMPANY PROPERTY.** On the date of the Executive's termination of employment with the Company for any reason (or at any time prior thereto at the Company's request), the Executive shall return all property belonging to the Company or its affiliates (including, but not limited to, any Company-provided laptops, computers, cell phones, wireless electronic mail devices or other equipment, or documents and property belonging to the Company). The Executive may retain the Executive's rolodex and similar address books provided that such items only include contact information. To the extent that the Executive is provided with a cell phone number by the Company during employment, the Company shall cooperate with the Executive in transferring such cell phone number to the Executive's individual name following the date of termination.

(e)**REFORMATION.** If it is determined by a court of competent jurisdiction in any state that any restriction in this Section 10 is excessive in duration or scope or is unreasonable or unenforceable under the laws of that state, it is the intention of the parties that such restriction may be modified or amended by the court to render it enforceable to the maximum extent permitted by the laws of that state.

(f)**TOLLING.** In the event of any violation of the provisions of this Section 10, the Executive acknowledges and agrees that the post-termination restrictions contained in this Section 10 shall be extended by a period of time equal to the period of such violation, it being the intention of the parties hereto that the running of the applicable post-termination restriction period shall be tolled during any period of such violation.

(g)**SURVIVAL OF PROVISIONS.** The obligations contained in Sections 10 and 11 hereof as well as those set forth in the Confidential Proprietary Rights and Non-Disclosure Agreement attached as Exhibit B (the "Confidentiality Agreement") shall survive the termination or expiration of the Employment Term and any termination of the Executive's employment with the Company and shall be fully enforceable thereafter.

11.COOPERATION. Upon the receipt of reasonable notice from the Company (including outside counsel), the Executive agrees that while employed by the Company and thereafter, the Executive will respond and provide information with regard to matters in which the Executive has knowledge as a result of the Executive's employment with the Company, and will provide reasonable assistance to the Company, its affiliates and their respective representatives in defense of any claims that may be made against the Company or its affiliates, and will assist the Company and its affiliates in the prosecution of any claims that may be made by the Company or its affiliates, to the extent that such claims may relate to the period of the Executive's employment with the Company. The Executive agrees to promptly inform the Company if the Executive becomes aware of any lawsuits involving such claims that may be filed or threatened against the Company or its affiliates. The Executive also agrees to promptly inform the Company (to the extent that the Executive is legally permitted to do so) if the Executive is asked to assist in any investigation of the Company or its affiliates (or their actions), regardless of whether a lawsuit or

other proceeding has then been filed against the Company or its affiliates with respect to such investigation, and shall not do so unless legally required. Upon presentation of appropriate documentation, the Company shall pay or reimburse the Executive for all reasonable out-of-pocket travel, duplicating or telephonic expenses incurred by the Executive in complying with this Section 11 and, in the event Executive is no longer receiving any compensation or benefits under this Agreement or as a Company employee, shall pay Executive a reasonable hourly rate for any work performed at Company's request.

12.EQUITABLE RELIEF AND OTHER REMEDIES. The Executive acknowledges and agrees that the Company's remedies at law for a breach or threatened breach of any of the provisions of Section 10 or Section 11 hereof would be inadequate and, in recognition of this fact, the Executive agrees that, in the event of such a breach or threatened breach, in addition to any remedies at la w, the Company, without posting any bond, shall be entitled to obtain equitable relief in the form of specific performance, a temporary restraining order, a temporary or permanent injunction or any other equitable remedy which may then be available. In the event that a court of competent jurisdiction or an arbitrator determines that Executive has violated Section 10 or Section 11 hereof, any severance being paid to the Executive pursuant to this Agreement or otherwise shall immediately cease, and any severance previously paid to the Executive (other than $1,000) shall be immediately repaid by the Executive to the Company.

13.NO ASSIGNMENTS. This Agreement is personal to each of the parties hereto. Except as provided in this Section 13 hereof, no party may assign or delegate any rights or obligations hereunder without first obtaining the written consent of the other party hereto. The Company may assign this Agreement to any successor to all or substantially all of the business and/or assets of the Company, provided that the Company shall require such successor to expressly assume and agree to perform this Agreement in the same manner and to the same extent that the Company would be required to perform it if no such succession had taken place. As used in this Agreement, "Company" shall mean the Company and any successor to its business and/or assets, which assumes and agrees to perform the duties and obligations of the Company under this Agreement by operation of law or otherwise.

14.NOTICE. For purposes of this Agreement, notices and all other communications provided for in this Agreement shall be in writing and shall be deemed to have been duly given (a) on the date of delivery, if delivered by hand, (b) on the date of transmission, if delivered by confirmed facsimile or electronic mail, (c) on the first business day following the date of deposit, if delivered by guaranteed overnight delivery service, or (d) on the fourth business day following the date delivered or mailed by United States registered or certified mail, return receipt requested, postage prepaid, addressed as follows:

If to the Executive:

At the address (or to the facsimile number) shown
on the records of the Company

If to the Company:
Sarepta Therapeutics, Inc.
215 First St.
Cambridge, MA 02142
Attention: Ty Howton, General Counsel

or to such other address as either party may have furnished to the other in writing in accordance herewith, except that notices of change of address shall be effective only upon receipt.

15.SECTION HEADINGS; INCONSISTENCY. The section headings used in this Agreement are included solely for convenience and shall not affect, or be used in connection with, the interpretation of this Agreement. In the event of any inconsistency between the terms of this Agreement and any form, award, plan or policy of the Company, the terms of this Agreement shall govern and control except in the case of any such form, award, plan or policy approved by the shareholders of the Company in which case the terms such form, award, plan or policy shall prevail.

16.SEVERABILITY. The provisions of this Agreement shall be deemed severable and the invalidity or unenforceability of any provision shall not affect the validity or enforceability of the other provisions hereof.

17.COUNTERPARTS. This Agreement may be executed in several counterparts, each of which shall be deemed to be an original but all of which together will constitute one and the same instrument.

18.ARBITRATION. Any dispute or controversy arising under or in connection with this Agreement or the Executive's employment with the Company, other than injunctive relief under Section 12 hereof, shall be settled exclusively by arbitration, conducted before a single arbitrator in Boston, Massachusetts (applying Massachusetts law) in accordance with the National Rules for the Resolution of Employment Disputes of the American Arbitration Association then in effect. The decision of the arbitrator will be final and binding upon the parties hereto. Judgment may be entered on the arbitrator's award in any court having jurisdiction. The parties acknowledge and agree that in connection with any such arbitration and regardless of outcome, (a) each party shall pay all of its own costs and expenses, including, without limitation, its own legal fees and expenses, and (b) the arbitration costs shall be borne entirely by the Company.

19.INDEMNIFICATION. The Company hereby agrees to indemnify Executive and hold Executive harmless to the fullest extent permitted by law against and in respect of any and all actions, suits, proceedings, claims, demands, judgments, costs, expenses (including advancement of reasonable attorney's fees), losses, and damages resulting from Executive's good faith performance of Executive's duties and obligations with the Company and the Company's affiliates. These obligations shall survive the expiration of the Employment Term and the termination of Executive's employment with the Company.

20.LIABILITY INSURANCE. The Company shall cover the Executive under directors' and officers' liability insurance both during and, while potential liability exists, after the term of this Agreement in the same amount and to the same extent as the Company covers its other officers and directors. These obligations shall survive the expiration of the Employment Term and the termination of Executive's employment with the Company.

21.MISCELLANEOUS. No provision of this Agreement may be modified, waived or discharged unless such waiver, modification or discharge is agreed to in writing and signed by the Executive and such officer or director as may be designated by the Board. No waiver by either party hereto at any time of any breach by the other party hereto of, or compliance with, any condition or provision of this Agreement to be performed by such other party shall be deemed a waiver of similar or dissimilar provisions or conditions at the same or at any prior or subsequent time. This Agreement, together with all exhibits hereto, the Confidentiality Agreement and the Severance Agreement, sets forth the entire agreement of the parties hereto in respect of the subject matter contained herein and supersedes any and all prior agreements or understandings between the Executive and the Company with respect to the subject matter hereof. No agreements or representations, oral or otherwise, express or implied, with respect to the subject matter hereof have been made by either party which are not expressly set forth in this Agreement. The validity, interpretation, construction and performance of this Agreement shall be governed by the laws of the Commonwealth of Massachusetts without regard to the choice of law principles thereof.

22.REPRESENTATIONS. The Executive represents and warrants to the Company that (a) the Executive has the legal right to enter into this Agreement and to perform all of the obligations on the Executive's part to be performed hereunder in accordance with its terms, and (b) the Executive is not a party to any agreement or understanding, written or oral, and is not subject to any restriction, which, in either case, could prevent the Executive from entering into this Agreement or performing all of the Executive's duties and obligations hereunder. In addition, the Executive acknowledges that the Executive is aware of Section 304 (Forfeiture of Certain Bonuses and Profits) of the Sarbanes-Oxley Act of 2002 and the right of the Company to be reimbursed for certain payments to the Executive in compliance with any policy Company may adopt in connection therewith.

23.LEGAL FEES. Within thirty (30) days upon presentation of appropriate documentation, the Company shall pay all reasonable and documented legal fees and related expenses incurred in connection with the drafting, negotiation and execution of this Agreement and other documents relating to equity arrangements, up to a maximum of $5,000.

24.TAX WITHHOLDING. The Company may withhold from any and all amounts payable under this Agreement such federal, state and local taxes as may be required to be withheld pursuant to any applicable law or regulation.

25.CODE SECTION 409A COMPLIANCE.

(a)The intent of the parties is that payments and benefits under this Agreement comply with, or be exempt from, Internal Revenue Code Section 409A and the regulations and guidance promulgated thereunder (collectively "Code Section 409A") and, accordingly, to the maximum extent permitted, this Agreement shall be interpreted to be in compliance therewith. If the Executive notifies the Company (with specificity as to the reason therefor) that the Executive believes that any provision of this Agreement (or of any award of compensation, including equity compensation or benefits) would cause the Executive to incur any additional tax or interest under Code Section 409A and the Company concurs with such belief or the Company (without any obligation whatsoever to do so) independently makes such determination, the Company shall, after consulting with the Executive, reform such provision to try to comply with Code Section 409A

through good faith modifications to the minimum extent reasonably appropriate to conform with Code Section 409A. To the extent that any provision hereof is modified in order to comply with Code Section 409A, such modification shall be made in good faith and shall, to the maximum extent reasonably possible, maintain the original intent and economic benefit to the Executive and the Company of the applicable provision without violating the provisions of Code Section 409A.

(b) A termination of employment shall not be deemed to have occurred for purposes of any provision of this Agreement providing for the payment of any amounts or benefits upon or following a termination of employment that are considered "nonqualified deferred compensation" under Code Section 409A unless such termination is also a "separation from service" within the meaning of Code Section 409A and, for purposes of any such provision of this Agreement, references to a "termination," "termination of employment" or like terms shall mean "separation from service." If the Executive is deemed on the date of termination to be a "specified employee" within the meaning of that term under Code Section 409A(a)(2)(B), then with regard to any payment that is considered non-qualified deferred compensation under Code Section 409A payable on account of a "separation from service," such payment or benefit shall be made or provided at the date which is the earlier of (i) the expiration of the six (6)-month period measured from the date of such "separation from service" of the Executive, and (ii) the date of the Executive's death (the "Delay Period"). Upon the expiration of the Delay Period, all payments and benefits delayed pursuant to this Section 25 (whether they would have otherwise been payable in a single sum or in installments in the absence of such delay) shall be paid or reimbursed to the Executive in a lump sum without interest, and any remaining payments and benefits due under this Agreement shall be paid or provided in accordance with the normal payment dates specified for them herein.

(c) With regard to any provision herein that provides for reimbursement of costs and expenses or in-kind benefits, except as permitted by Code Section 409A, (i) the right to reimbursement or in-kind benefits shall not be subject to liquidation or exchange for another benefit, (ii) the amount of expenses eligible for reimbursement, or in-kind benefits, provided during any taxable year shall not affect the expenses eligible for reimbursement, or in-kind benefits to be provided, in any other taxable year, provided that the foregoing clause (ii) shall not be violated without regard to expenses reimbursed under any arrangement covered by Internal Revenue Code Section 105(b) solely because such expenses are subject to a limit related to the period the arrangement is in effect and (iii) such payments shall be made on or before the last day of Executive's taxable year following the taxable year in which the expense occurred.

(d) For purposes of Code Section 409A, the Executive's right to receive any installment payments pursuant to this Agreement shall be treated as a right to receive a series of separate and distinct payments. In no event may the Executive, directly or indirectly, designate the calendar year of any payment to be made under this Agreement that is considered nonqualified deferred compensation. In no event shall the timing of Executive's execution of the Release, directly or indirectly, result in the Executive designating the calendar year of payment, and if a payment that is subject to execution of the Release could be made in more than one taxable year, payment shall be made in the later taxable year.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first written above.

COMPANY

By:/s/ M. Kathleen Behrens
Name:M. Kathleen Behrens
Title:Chairwoman of the Board of Directors

EXECUTIVE

By:/s/ Edward M. Kaye, M.D.
Name: Edward M. Kaye, M.D.

EXHIBIT A

[RELEASE OF CLAIMS]

EXHIBIT B

[CONFIDENTIAL PROPRIETARY RIGHTS AND NON-DISCLOSURE AGREEMENT]

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

Exhibit 10.2

LICENSE AND COLLABORATION AGREEMENT

by and between

SUMMIT (OXFORD) LTD

and

SAREPTA THERAPEUTICS, INC.

Dated as of October 3, 2016

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

TABLE OF CONTENTS

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

EXHIBIT & SCHEDULES

Exhibit A	Development Plan and Budget (to be attached upon JSC approval)
Schedule 1.34	University of Oxford Option Agreement
Schedule 1.64	Option Data Package
Schedule 1.89	Summit Patent Rights
Schedule 6.2	Supply Agreement Terms
Schedule 9.2.3	Joint Press Release

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

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
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)

DEFINITION:	SECTION:
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 [**] 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, if [**] 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 suc h 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 term ination 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 [**] 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]

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

IN WITNESS WHEREOF, the Parties have executed this Agreement as of the Effective Date.

SAREPTA THERAPEUTICS, INC.	**SUMMIT (OXFORD) LTD**
BY:/s/ Edward M. Kaye, M.D.	BY:/s/ Glyn O. Edwards
NAME: Edward M. Kaye, M.D.	NAME: Glyn O. Edwards
TITLE: President and Chief Executive Officer	TITLE: Chief Executive Officer

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

EXHIBIT A

INITIAL DEVELOPMENT PLAN

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

SCHEDULE 1.34

EXISTING SUMMIT IN-LICENSES

University of Oxford Option Agreement

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

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. [**]

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

SCHEDULE 1.89

SUMMIT PATENT RIGHTS

[**]

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

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.

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

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.

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

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

For Sarepta Therapeutics:

Sarepta
Ian Estepan Tel: 617-274-4052
iestepan@sarepta.com

W2O Group
Brian Reid Tel: 212-257-6725
breid@w2ogroup.com

For Summit:

Summit
Glyn Edwards / Richard Pye (UK office) Tel: +44 (0)1235 443 951
Erik Ostrowski / Michelle Avery (US office) +1 617 225 4455

Cairn Financial Advisers LLP
(Nominated Adviser)
Liam Murray / Tony Rawlinson Tel: +44 (0)20 77148 7900

[**] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Commission.

N+1 Singer (Broker)
Aubrey Powell / Jen Boorer — Tel: +44 (0)20 7496 3000

MacDougall Biomedical Communications
(US media contact)
Chris Erdman / Karen Sharma — Tel: +1 781 235 3060
cerdman@macbiocom.com
ksharma@macbiocom.com

Consilium Strategic Communications
(Financial public relations, UK)
Mary-Jane Elliott / Sue Stuart /
Jessica Hodgson / Lindsey Neville — Tel: +44 (0)20 3709 5700
Summit@consilium-comms.com

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 31.1

CERTIFICATION

I, Edward Kaye, MD, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc., (the "Registrant");

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 Registrant as of, and for, the periods presented in this report;

4. The Registrant'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 Registrant 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 Registrant, 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 Registrant'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 Registrant's internal control over financial reporting that occurred during the Registrant's most recent fiscal quarter (the Registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the Registrant's internal control over financial reporting; and

5. The Registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Registrant's auditors and the audit committee of the Registrant'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 Registrant'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 Registrant's internal control over financial reporting.

November 7, 2016

/s/ Edward Kaye, MD
Edward Kaye, MD
President, Chief Executive Officer and Chief Medical Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION

I, Sandesh Mahatme, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc., (the "Registrant");

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 Registrant as of, and for, the periods presented in this report;

4. The Registrant'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 Registrant 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 Registrant, 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 Registrant'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 Registrant's internal control over financial reporting that occurred during the Registrant's most recent fiscal quarter (the Registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the Registrant's internal control over financial reporting; and

5. The Registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Registrant's auditors and the audit committee of the Registrant'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 Registrant'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 Registrant's internal control over financial reporting.

November 7, 2016

/s/ Sandesh Mahatme

Sandesh Mahatme
Senior Vice President, Chief Financial Officer
(Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Edward Kaye, MD, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended September 30, 2016, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

November 7, 2016

/s/ Edward Kaye, MD

Edward Kaye, MD
President, Chief Executive Officer and Chief Medical Officer
(Principal Executive Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.

Exhibit 32.2

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Sandesh Mahatme, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended September 30, 2016, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

November 7, 2016

/s/ Sandesh Mahatme
Sandesh Mahatme
Senior Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.