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 March 31, 2017

OR

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

For the transition period from to

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	☐
Emerging growth company	☐		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

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	**54,955,063**
(Class)	**(Outstanding as of April 28, 2017)**

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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 March 31, 2017	As of December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 274,723	$ 122,420
Short-term investments	115,590	195,425
Accounts receivable	12,278	5,228
Inventory	30,445	12,813
Restricted investment	—	10,695
Asset held for sale	1,529	—
Other current assets	22,742	26,895
Total current assets	457,307	373,476
Restricted cash and investments	784	784
Property and equipment, net of accumulated depreciation of $31,488 and $30,346 as of March 31, 2017 and December 31, 2016, respectively	38,412	37,801
Intangible assets, net of accumulated amortization of $2,758 and $3,134 as of March 31, 2017 and December 31, 2016, respectively	7,684	8,076
Other non-current assets	7,346	3,967
Total assets	$ 511,533	$ 424,104
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 27,446	$ 29,690
Accrued expenses	31,157	31,016
Current portion of long-term debt	11,346	10,108
Deferred revenue	3,303	3,303
Other current liabilities	1,377	1,305
Total current liabilities	74,629	75,422
Long-term debt	2,343	6,042
Deferred rent and other	5,419	5,949
Total liabilities	82,391	87,413
Commitments and contingencies (Note 15)		
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; 54,940,604 and 54,759,234 issued and outstanding at March 31, 2017 and December 31, 2016, respectively	5	5
Additional paid-in capital	1,511,422	1,503,126
Accumulated other comprehensive loss	(55)	(120)
Accumulated deficit	(1,082,230)	(1,166,320)
Total stockholders' equity	429,142	336,691
Total liabilities and stockholders' equity	$ 511,533	$ 424,104

See accompanying notes to unaudited condensed consolidated financial statements.

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

	For the Three Months Ended March 31,	
	2017	2016
Revenues:		
Product, net	$ 16,342	$ —
Total revenues	16,342	—
Costs and expenses:		
Cost of sales	252	—
Research and development	29,119	38,826
Selling, General and administrative	26,216	20,876
Total cost and expenses	55,587	59,702
Operating loss	(39,245)	(59,702)
Other income (loss):		
Gain from sale of Priority Review Voucher	125,000	—
Interest income (expense) and other, net	335	(68)
Total other income (loss)	125,335	(68)
Income (loss) before income tax expense	86,090	(59,770)
Income tax expense	2,000	—
Net income (loss)	84,090	(59,770)
Other comprehensive income:		
Unrealized gain on short-term securities - available-for-sale	65	106
Total other comprehensive income	65	106
Comprehensive income (loss)	$ 84,155	$ (59,664)
Net income (loss) per share:		
Basic earnings (loss) per share	$ 1.53	$ (1.31)
Diluted earnings (loss) per share	$ 1.50	$ (1.31)
Weighted average number of shares of common stock used in calculating:		
Basic earnings (loss) per share	54,850	45,697
Diluted earnings (loss) per share	56,012	45,697

See accompanying notes to unaudited condensed consolidated financial statements.

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

	For the Three Months Ended March 31,	
	2017	2016
Cash flows from operating activities:		
Net income (loss)	$ 84,090	$ (59,770)
Adjustments to reconcile net income (loss) to cash flows from operating activities:		
Gain from sale of Priority Review Voucher	(125,000)	—
Depreciation and amortization	1,637	1,397
Amortization of (discount) premium on available-for-sale securities and non-cash interest	(18)	242
Loss on abandonment of patents	485	15
Stock-based compensation	5,712	6,835
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(7,050)	(13)
Net increase in inventory	(17,632)	—
Net decrease (increase) in other assets	774	(3,180)
Net decrease in accounts payable, accrued expenses, deferred revenue and other liabilities	(886)	(6,214)
Net cash used in operating activities	(57,888)	(60,688)
Cash flows from investing activities:		
Purchase of property and equipment	(4,465)	(1,168)
Purchase of intangible assets	(1,245)	(410)
Proceeds from sale of Priority Review Voucher	125,000	—
Maturity of restricted investment	10,695	—
Maturity of available-for-sale securities	80,000	21,000
Net cash provided by investing activities	209,985	19,422
Cash flows from financing activities:		
Repayments of long-term debt and notes payable	(2,543)	(2,525)
Proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program	2,749	1,488
Net cash provided by (used in) financing activities	206	(1,037)
Increase (decrease) in cash and cash equivalents	152,303	(42,303)
Cash, cash equivalents and restricted cash:		
Beginning of period	122,556	80,439
End of period	274,859	38,136
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 291	$ 409
Supplemental schedule of non-cash investing activities and financing activities:		
Shares withheld for taxes	$ 165	$ 44
Accrual for debt issuance costs related to the senior secured term loan	$ 400	$ 400
Intangible assets included in accrued expenses	$ 179	$ 192
Property and equipment included in accrued expenses	$ 330	$ 19

See accompanying notes to unaudited condensed consolidated financial statements.

1. 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. In November 2016, the Company submitted a marketing authorization application ("MAA") for eteplirsen to the European Medicine Agency ("EMA") and the application was validated in December 2016.

As of March 31, 2017, the Company had approximately $391.1 million of cash, cash equivalents and investments, consisting of $274.7 million of cash and cash equivalents, $115.6 million of short-term investments and $0.8 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of March 31, 2017 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, seek additional government funding and establish collaborations with or license its technology to other companies.

2. SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

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 ("U.S. GAAP"), reflect the accounts of Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients for the treatment of rare neuromuscular diseases.

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 revenue recognition, inventory, the valuation of stock-based awards, research and development expenses and income tax.

Concentration of Credit Risk

Financial instruments which potentially subject the Company to concentrations of credit risk consist of accounts receivable from customers and cash, cash equivalent and investments held at financial institutions.

For the three months ended March 31, 2017, the majority of the Company's accounts receivable arose from product sales in the U.S. and all customers have standard payment terms which generally require payment within 30 to 45 days. Three individual customers accounted for 60%, 23% and 17% of net product revenues and 61%, 22% and 17% of accounts receivable from product sales, respectively. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profile. As of March 31, 2017, the Company believes that such customers are of high credit quality.

As of March 31, 2017, the Company's money market funds and short-term investments were concentrated at a single financial institution, which potentially exposes the Company to credit risks. However, the Company does not believe that there is significant risk of non-performance by the financial institution.

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, 2016.

In November 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-18, "Statement of Cash Flows: Restricted Cash". The amendments in this update requires amounts generally described as restricted cash and restricted cash equivalents be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU No. 2016-18 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. The Company elected to early adopt this guidance as of January 1, 2017. This guidance was applied using a retrospective transition method for each period presented.

There have not been any other material changes to the Company's accounting policies as of March 31, 2017.

Recent Accounting Pronouncements

In August 2016, the FASB issued 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 March 31, 2017, 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 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. In transition, lessees and lessors are required to recognize and measure leases at the beginning of the earliest period presented using a modified retrospective approach. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The adoption of this standard is expected to have an impact on the amount of the Company's assets and liabilities. As of March 31, 2017, 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 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 was originally intended to be 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*". In December 2016, the FASB issued ASU No. 2016-20, "*Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers",* which amends certain narrow aspects of the guidance issued in ASU 2014-09 including guidance related to the disclosure of remaining performance obligations and prior-period performance obligations, as well as other amendments to the guidance on loan guarantee fees, contract costs, refund liabilities, advertising costs and the clarification of certain examples. These three amendments will be effective upon adoption of Topic 606. As of March 31, 2017, 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. GAIN FROM SALE OF PRIORITY REVIEW VOUCHER

On February 21, 2017, the Company entered into an agreement with Gilead Sciences, Inc. ("Gilead") to sell the Company's Rare Pediatric Disease Priority Review Voucher ("PRV"). The Company received the PRV when EXONDYS 51 was approved by the FDA for the treatment of patients with DMD amenable to exon 51 skipping. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, on March 30, 2017, the Company completed its sale of the PRV to a subsidiary of Gilead. Pursuant to the Agreement, the subsidiary of Gilead paid the Company $125.0 million, which was recorded as a gain from sale of the PRV as it did not have a carrying value at the time of the sale.

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 March 31, 2017			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 81,286	$ 81,286	$ —	$ —
Commercial paper	29,435	—	29,435	—
Government and government agency bonds	65,329	—	65,329	—
Corporate bonds	20,826	—	20,826	—
Certificates of deposit	684	684	—	—
Total assets	$ 197,560	$ 81,970	$ 115,590	$ —

	Fair Value Measurement as of December 31, 2016			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 1,147	$ 1,147	$ —	$ —
Commercial paper	69,304	—	69,304	—
Government and government agency bonds	105,287	—	105,287	—
Corporate bonds	20,834	—	20,834	—
Certificates of deposit	11,343	11,343	—	—
Total assets	$ 207,915	$ 12,490	$ 195,425	$ —

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 March 31, 2017.

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. The weighted average maturity of the Company's available-for-sale securities as of March 31, 2017 and December 31, 2016 was approximately seven and four months, respectively.

The following tables summarize the Company's cash, cash equivalents and short-term investments for each of the periods indicated:

	As of March 31, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 274,723	$ —	$ —	$ 274,723
Commercial paper	29,450	—	(15)	29,435
Government and government agency bonds	65,361	—	(32)	65,329
Corporate bonds	20,834	—	(8)	20,826
Total assets	$ 390,368	$ —	$ (55)	$ 390,313
As reported:				
Cash and cash equivalents	$ 274,723	$ —	$ —	$ 274,723
Short-term investments	115,645	—	(55)	115,590
Total assets	$ 390,368	$ —	$ (55)	$ 390,313

	As of December 31, 2016			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 122,420	$ —	$ —	$ 122,420
Commercial paper	69,355	—	(51)	69,304
Government and government agency bonds	105,340	—	(53)	105,287
Corporate bonds	20,850	—	(16)	20,834
Total assets	$ 317,965	$ —	$ (120)	$ 317,845
As reported:				
Cash and cash equivalents	$ 122,420	$ —	$ —	$ 122,420
Short-term investments	195,545	—	(120)	195,425
Total assets	$ 317,965	$ —	$ (120)	$ 317,845

6. ACCOUNTS RECEIVABLE AND RESERVES FOR PRODUCT SALES

The Company's accounts receivable arise from product sales, government research contracts and other grants. They are generally stated at the invoiced amount and do not bear interest.

The accounts receivable from product sales represents receivables due from the Company's specialty distributor and specialty pharmacies. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profiles. The Company provides reserves against trade receivables for estimated losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are written-off against the established reserve. As of March 31, 2017, the credit profiles for the Company's customers are deemed to be in good standing and write-offs of accounts receivable are not considered necessary. Historically, no accounts receivable amounts related to government research contracts and other grants have been written off and, thus, an allowance for doubtful accounts receivable related to government research contracts and other grants is not considered necessary.

The following table summarizes the components of the Company's accounts receivable for the periods indicated:

	As of March 31, 2017	As of December 31, 2016
	(in thousands)	
Trade receivables	$ 11,349	$ 4,002
Unbilled receivables	929	1,226
Total accounts receivable	$ 12,278	$ 5,228

The balance for unbilled receivables for both periods presented is subject to government audit and will not be collected until the completion of the audit. The decrease in unbilled receivables is related to contract finalization and subsequent collection of the European Union SKIP-NMD Agreement related to the Company's exon 53 product candidate.

The following table summarizes an analysis of the change in reserves for discounts and allowances for the periods indicated:

	Chargebacks	Rebates	Other Accruals	Total
		(in thousands)		
Balance, as of December 31, 2016	$ 1	$ 238	$ 67	$ 306
Provision	256	608	132	996
Payments/credits	(152)	(88)	(82)	(322)
Balance, as of March 31, 2017	$ 105	$ 758	$ 117	$ 980

The following table summarizes the total reserves above included in the Company's unaudited condensed consolidated balance sheets for the periods indicated:

	As of March 31, 2017	As of December 31, 2016
	(in thousands)	
Reduction to accounts receivable	$ 105	$ 1
Component of accrued expenses	875	305
Total reserves	$ 980	$ 306

7. 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 following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. EXONDYS 51 which may 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 March 31, 2017	As of December 31, 2016
	(in thousands)	
Raw materials	$ 24,898	$ 9,531
Work in progress	5,433	3,175
Finished goods	114	107
Total inventory	$ 30,445	$ 12,813

8. ASSET HELD FOR SALE

The Company owns a facility located at 1749 SW Airport Avenue, Corvallis, OR ("Airport Facility"). The Airport Facility was previously leased to an unrelated third party. In July 2016, the third party lessee terminated the lease and vacated the facility. It has been unoccupied since then. The Company has set up a program and is actively marketing the Airport Facility. As of March 31, 2017, the Airport Facility with net book value of approximately $1.5 million was reclassified as an asset held for sale which is presented as a component of current assets.

9. 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 March 31, 2017	As of December 31, 2016
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 18,451	$ 23,604
Prepaid clinical expenses	1,822	1,163
Other prepaids	1,513	1,214
Other	956	914
Total other current assets	$ 22,742	$ 26,895

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

	As of March 31, 2017	As of December 31, 2016
	(in thousands)	
Prepaid clinical expenses	$ 3,891	$ 3,725
Manufacturing-related deposits	3,213	—
Other	242	242
Total other non-current assets	$ 7,346	$ 3,967

10. ACCRUED EXPENSES

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

	As of March 31, 2017	As of December 31, 2016
	(in thousands)	
Accrued clinical and preclinical costs	$ 11,134	$ 10,033
Accrued contract manufacturing costs	5,736	4,673
Accrued employee compensation costs	5,177	8,748
Accrued professional fees	3,759	2,799
Product revenue related reserve	875	305
Accrued research costs	441	1,186
Other	4,035	3,272
Total accrued expenses	$ 31,157	$ 31,016

11. RESTRUCTURING

In March 2016, the Company announced a long-term plan ("Corvallis plan") to consolidate all of the Company's operations to Massachusetts as part of a strategic plan to increase operational efficiency. As part of the consolidation, research activities and some employees have transitioned to the Company's facilities in Andover and Cambridge, Massachusetts. As of March 31, 2017, the relocations and terminations were substantially completed.

In December 2016, the second floor of the two floors at the Corvallis facility was vacated and closed and made available for sub-leasing. The first floor of the facility continues to be used by the Company's employees. As of March 31, 2017, the Company continues to be obligated to make $6.0 million of minimum lease payments and certain other contractual maintenance costs for the whole facility.

For the three months ended March 31, 2017, the Company recognized $0.2 million of restructuring expenses, the majority of which related to relocation of equipment from Oregon to Massachusetts. For the three months ended March 31, 2016, $0.5 million of restructuring expenses related to workforce reduction.

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

	For the Three Months Ended March 31, 2017			For the Three Months Ended March 31, 2016		
			(in thousands)			
	Cash	Non-cash	Total	Cash	Non-cash	Total
Research and development	$ 70	$ —	$ 70	$ 357	$ 145	$502
General and administration	166	—	166	31	—	31
Total restructuring expenses	$ 236	$ —	$ 236	$ 388	$ 145	$533

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

	For the Quarter Ended March 31, 2017	For the Year Ended December 31, 2016
	(in thousands)	
Restructuring reserve beginning balance	$ 1,588	$ —
Restructuring expenses incurred during the period	236	3,651
Amounts paid during the period	(394)	(2,063)
Restructuring reserve ending balance	$ 1,430	$ 1,588

12. STOCK-BASED COMPENSATION

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

	For the Three Months Ended March 31,			
	2017		2016	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	877,492	$ 16.30	1,205,776	$ 11.92
Restricted stock units (1)	161,029	$ 32.63	—	$ —
Restricted stock awards	6,500	$ 31.06	25,775	$ 13.71

(1) The Company granted certain executives 156,029 restricted stock units ("RSU") with certain sales target and regulatory milestones. As of March 31, 2017, the performance conditions of these RSUs were not probable of being achieved. If and when deemed probable that such performance milestones may be achieved within the required time frame, the Company may recognize up to $5.1 million of stock-based compensation related to these grants. The remaining RSUs are service-based awards granted to the members of the board of directors.

Stock-based Compensation Expense

For the three months ended March 31, 2017 and 2016, total stock-based compensation expense was $5.7 million and $6.7 million, respectively. 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 March 31,	
	2017	2016
	(in thousands)	
Research and development	1,874	2,449
General and administrative	3,838	4,241
Total stock-based compensation expense	$ 5,712	$ 6,690

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 March 31,	
	2017	2016
	(in thousands)	
Stock options	$ 5,038	$ 5,698
Restricted stock awards/units	204	184
Stock appreciation rights	—	115
Employee stock purchase plan	470	693
Total stock-based compensation expense	$ 5,712	$ 6,690

13. INCOME TAXES

The Company's tax provision for interim periods is typically determined using an estimate of its annual effective tax rate, adjusted for discrete items arising in that quarter. In each quarter, the Company updates its estimate of the annual effective tax rate, and if the estimated annual tax rate changes, the Company makes a cumulative adjustment in that quarter. The Company computed its tax provision for the three months ended March 31, 2017 based upon the year-to-date effective tax rate as opposed to an estimated annual effective tax rate. The Company concluded that the year-to-date effective tax rate is the most appropriate method to use for the three months ended March 31, 2017, given a reliable estimate of the annual effective tax rate cannot be made.

For the three months ended March 31, 2017, the Company recorded a provision for income taxes of $2.0 million, and for the three months ended March 31, 2016, the Company did not record an income tax provision or benefit, resulting in an effective tax rate of 2.3% and 0%, respectively. Increase in the income tax expense as of March 31, 2017 as compared to the balance as of December 31, 2016 is due to additional state and federal income taxes payable as a result of the increase in the amount of income before income taxes. The increase in income is primarily attributable to the gain on the sale of the Company's PRV to Gilead for $125.0 million in cash during the period ended March 31, 2017.

14. NET EARNINGS (LOSS) PER SHARE

Basic net earnings (loss) per share is computed by dividing net income (loss) by the weighted-average number of shares of common stock outstanding. Diluted net earnings (loss) per share is computed by dividing net income (loss) by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. For the three months ended March 31, 2016, there was 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 March 31,	
	2017	2016
	(in thousands, except per share amounts)	
Net income (loss)	$ 84,090	$ (59,770)
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	54,850	45,697
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	1,162	—
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	56,012	45,697
Net income (loss) per share:		
Basic earnings (loss) per share	$ 1.53	$ (1.31)
Diluted earnings (loss) per share	$ 1.50	$ (1.31)

* For the three months ended March 31, 2017, out of money stock options, unvested performance-based RSUs and restricted stock awards whose performance milestones were not achieved and potentially issuable common stock for ESPP to purchase approximately 3.4 million were excluded from the net earnings per share calculation as their effect would have been anti-dilutive. For the three months ended March 31, 2016, stock options, RSAs and SARs to purchase 7.9 million were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

15. 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. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (Corban v. Sarepta, et. al., No. 14-cv-10201) by order of the court on June 23, 2014. Plaintiffs' consolidated amended complaint, filed on July 21, 2014, asserted violations of Section 10(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act") and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian, Sandy Mahatme, and Ed Kaye ("Individual Defendants," and collectively with the Company, the "Corban Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Corban Defendants made material misrepresentations or omissions during the putative class period of July 24, 2013 through November 12, 2013, regarding a data set for a Phase 2b study of eteplirsen and the likelihood of the FDA accepting the Company's new drug application for eteplirsen for review based on that data set. Plaintiffs sought compensatory damages and fees. On August 18, 2014, the Corban Defendants filed a motion to dismiss, which the Court granted on March 31, 2015. Plaintiffs subsequently sought leave to file a second amended complaint, which the Corban Defendants opposed. On September 2, 2015, the Court denied Plaintiffs' motion for leave to amend as futile. Plaintiffs filed a notice of appeal on September 29, 2015, seeking review of the Court's March 31, 2015 order dismissing the case and the Court's September 2, 2015 order denying leave to amend. On January 27, 2016, Plaintiffs filed in the district court a motion for relief from judgment pursuant to Federal Rule of Civil Procedure 60(b)(2), arguing that the FDA Briefing Document published on or about January 15, 2016, was material and would have changed the Court's ruling. On February 26, 2016, the First Circuit stayed the appeal pending the district court's ruling on the 60(b)(2) motion. Defendants opposed the 60(b)(2) motion, and on April 21, 2016, the Court denied Plaintiffs' motion for relief from judgment. On May 19, 2016, Plaintiffs filed a motion to alter or amend the April 21, 2016 order pursuant to Federal Rule of Civil Procedure 59(e). On May 20, 2016, the Court denied Plaintiffs' motion, and Plaintiffs filed a notice of appeal of the Court's April 21, 2016 denial of their 60(b)(2) motion and May 20, 2016 denial of their 59(e) motion. On June 13, 2016, the First Circuit granted Plaintiffs' motion to consolidate the two appeals. Oral argument took place on March 7, 2017. A decision has not yet been issued by the First Circuit. An estimate of the possible loss or range of loss cannot be made at this time.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 styled William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Christopher Garabedian, and Sandesh Mahatme (Kader v. Sarepta et.al 1:14-cv-14318). On March 20, 2015, Plaintiffs filed an amended complaint asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian and Sandy Mahatme ("Individual Defendants," and collectively with the Company, the "Kader Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Kader 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. Plaintiffs sought compensatory damages and fees. The Kader Defendants moved to dismiss the amended complaint on May 11, 2015. On April 5, 2016, following oral argument on March 29, 2016, the Court granted Defendants' motion to dismiss. On April 8, 2016, Lead Plaintiffs filed a motion for leave to file an amended complaint, which Defendants opposed. On January 6, 2017, the Court denied Plaintiffs' motion for leave to amend and dismissed the case. Plaintiffs filed a notice of appeal on February 3, 2017. A briefing schedule was set on March 13, 2017. Appellants' brief was filed April 24, 2017. Appellee's brief is due May 24, 2017. 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., 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, a derivative suit was filed 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., v. Behrens et al., No. 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 through the date of the complaint. 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. v. 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 Chief Executive Officer, 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. An estimate of the possible loss or range of loss cannot be made at this time.

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, 2016 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:

- *our ability to achieve a successful commercial launch of EXONDYS 51 in the U.S., including through executing our plans to hire additional personnel, increase awareness on the importance of genetic testing and knowing / understanding DMD mutations, and identify and address procedural barriers for patients to obtain therapy such as payor reimbursement challenges, maintaining the marketing, distribution and supply infrastructure we have built for EXONDYS 51 and our expectations regarding the timing, costs, and investments associated with these activities;*

- *our ability to obtain full approval of eteplirsen (i) in the U.S., which is dependent on our ability to complete to the United States Food and Drug Administration's ("FDA") satisfaction our post-marketing requirements and commitments, (ii) in the EU, which is dependent on our ability to successfully navigate the EU drug approval process and (iii) in other parts of the world that we may target;*

- *the potential acceptance of EXONDYS 51, and our product candidates if they receive regulatory approval, in the marketplace and the accuracy of our projections regarding the market size in each of the jurisdictions that we target;*

- *our ability to further secure long term supply of EXONDYS 51 and our product candidates, including by securing supply of subunits, drug substance Active Pharmaceutical Ingredients ("APIs") and drug product, to satisfy our planned commercial and clinical needs by, among other things, negotiating and entering into additional commercial and supply agreements, and further scaling up manufacturing using appropriate techniques to synthesize and purify our product candidates to meet regulatory, Company quality control and other applicable requirements;*

- *our expectations regarding our ability to successfully conduct or accelerate research, development, pre-clinical, clinical and post-approval trials, and our expectations regarding the timing, design and results of such trials, including their potential consistency with prior results, as well as data and analyses relating to the safety profile and potential clinical benefits of EXONDYS 51 and our product candidates;*

- *our success in advancing the development of our follow-on exon-skipping drug candidates targeting DMD and further exploring potential funding, collaborations and other opportunities to support such development;*

- *the potential and advancement of our phosphorodiamidate morpholino oligomer ("PMO") chemistries, our peptide-conjugated PMO ("PPMO") chemistries, our other PMO-based chemistries, and our other technologies to treat Duchenne muscular dystrophy ("DMD") and other diseases and therapeutic areas that we target;*

- *our ability to successfully expand the global footprint of eteplirsen, including through obtaining an approval from the European Medicines Agency ("EMA") in the EU and other jurisdictions where regulatory approval has not been obtained, establishing early access programs in other parts of the world, building the internal infrastructure for commercialization and ensuring commercial supply to support these plans, and our expectations regarding the EMA review process, including timing and the factors that will impact the EMA's evaluation and decision;*

- *the impact of regulations and 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;*

- *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 factors such as successfully establishing and maintaining the appropriate Company infrastructure necessary to support the Company's research, development and commercialization efforts;*

- *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;*

- *our ability to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities for specific molecular targets or selected disease indications and our ability to selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements;*

- *our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter 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 and continued commercialization, where authorized, of EXONDYS 51 and 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 Office (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 interference decisions regarding our patents and patent applications and those held by BioMarin Pharmaceutical, Inc., ("BioMarin") relating to exon 51, including EXONDYS 51, and exon 53, including SRP-4053, and our expectations regarding the impact of any appeal decisions in connection with these interferences on our business plans, including our commercialization for EXONDYS 51 and, if authorized, SRP-4053;*

- *the impact of any consequences of the interference decisions and ongoing appeals including the final refusal of BioMarin claims in the exon 53 and exon 51 composition of matter interferences, the cancellation of our patent in the exon 51 method of use interference, and the narrow claim BioMarin was allowed to pursue as a result of the exon 53 interference decision;*

- *the 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 appeals of Interference Nos. 106,007, 106,008 and 106,013, any of which could result in an infringement claim against us if the BioMarin patent applications subject to the appeals are ultimately granted;*

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

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

- *our ability to raise additional funds to support our business plans and strategies, including business development, and the impact of our credit and security agreement with MidCap Financial on our financial condition and future operations;*

- *our expectations relating to potential funding from government and other sources for the development of some of our product candidates;*

- *the timing and outcomes of ongoing interference proceedings and related appeals, and 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;*

- *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 exon-skipping drug candidates targeting DMD. 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. We commenced shipments of EXONDYS 51 to customers at the end of the third quarter of 2016. Additionally, we submitted a Marketing Authorization Application ("MAA") for eteplirsen to the EMA in November 2016 and the application was validated in December 2016.

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.

The original PMO structure and variations of this structure referred to herein (collectively "PMO-based") are central to our proprietary chemistry platform. Our next generation PMO-based chemistries include PPMO, PMO-X® and PMO*plus*®. 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-based compounds have demonstrated inhibition of mRNA translation and alteration of pre-mRNA splicing. 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 platform may represent a significant improvement over other RNA-targeted technologies. In addition, 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.

PPMO, our next generation chemistry, features covalent attachment of a cell-penetrating peptide to a PMO with the goal of enhanced cellular delivery into the cytosol and the nucleus. Based on our in-vivo pre-clinical research to date, we believe our proprietary class of PPMO compounds demonstrate an increase in dystrophin production and a more durable response compared to PMO, as well as a favorable tolerability profile in non-human primates. In these in-vivo pre-clinical studies, we also observed exon-skipping in skeletal, cardiac and smooth muscle. If additional studies provide consistent data with our studies to date, we believe that the use of PPMO could require less frequent dosing than PMO and could potentially be tailored to reach other organs. We are in the process of conducting IND-enabling GLP toxicology studies, and we are targeting filing an investigation new drug ("IND") application before year-end in 2017 for PPMO ("SRP-5051") DMD exon 51.

We are in the process of conducting, starting, or planning several studies in the U.S. and Europe for EXONDYS 51 and other product candidates designed to skip exons 45 and 53 ("SRP-4045" and "SRP-4053", respectively). These are comprised of:

(i) studies we are currently conducting to further evaluate EXONDYS 51, including an open label extension of our Phase 2b study, 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 EU regulatory requirements for IND applications and MAAs, respectively (e.g. a Phase 2 study on participants between the ages of six months and four years in connection with our PIP in the EU, and two additional Phase 1 studies);

(iii) studies we are planning to fulfill for our post-marketing FDA requirements/commitments for EXONDYS 51, including studies regarding SRP-4045 and/or SRP-4053;
(iv) a randomized, double-blind dose-ranging study that we completed for SRP-4045 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 SRP-4053 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 SRP-4045 and SRP 4053, which has begun enrolling patients in the U.S. and for which we plan to have sites in the EU, Israel and Canada.
(vii) additional Phase 1 studies we are planning to initiate for SRP-4053 and SRP-4045.

We believe we have developed proprietary state-of-the-art manufacturing and techniques that allow synthesis and purification of our product candidates to support both 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 production of certain of our product candidates and their components. We currently do not have any of our own internal manufacturing capabilities to produce our product and product candidates for commercial and/or clinical use.

As of March 31, 2017, we had approximately $391.1 million of cash, cash equivalents and investments, consisting of $274.7 million of cash and cash equivalents, $115.6 million of short-term investments and $0.8 million 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.

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, the risks associated with government sponsored programs and the complex regulatory environment in which we operate. We may never achieve significant revenue or profitable operations.

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;
- inventory;
- 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, 2016.

Results of Operations for the Three Months Ended March 31, 2017 and 2016

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

	For the Three Months Ended March 31,			
	2017	2016	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$ 16,342	$ —	$ 16,342	NA
Total revenues	16,342	—	16,342	NA
Costs and expenses:				
Cost of sales	252	—	252	NA
Research and development	29,119	38,826	(9,707)	(25)%
General and administrative	26,216	20,876	5,340	26%
Total cost and expenses	55,587	59,702	(4,115)	(7)%
Operating loss	(39,245)	(59,702)	20,457	(34)%
Other income(loss):				
Gain from sale of Priority Review Voucher	125,000	—	125,000	NA
Interest income (expense) and other, net	335	(68)	403	(593)%
Income (loss) before income tax expense	86,090	(59,770)	145,860	(244)%
Income tax expense	2,000	—	2,000	NA
Net income (loss)	$ 84,090	$ (59,770)	$ 143,860	(241)%
Net income (loss) per share:				
Basic earnings (loss) per share	$ 1.53	$ (1.31)	$ 2.84	(217)%
Diluted earnings (loss) per share	$ 1.50	$ (1.31)	$ 2.81	(215)%
Weighted average number of shares of common stock used in calculating:				
Basic earnings (loss) per share	54,850	45,697		
Diluted earnings (loss) per share	56,012	45,697		

Revenues

We record product revenues net of applicable discounts and allowances which include Medicaid rebates, Public Health Services chargebacks and co-pays. Reserves established for these discounts and allowances are classified as reductions of accounts receivable (if the amount is payable to our customer) or a liability (if the amount is payable to a party other than our customer). These reserves are based on estimates of the amounts earned or to be claimed on the related sales. Our estimates take into consideration current contractual and statutory requirements. Actual amounts may ultimately differ from our estimates. If actual results are different from our estimates, we adjust these estimates, which will have an effect on earnings in the period of adjustment. Product revenues, net reflect the commercial launch of EXONDYS 51 in the U.S. in September 2016.

Cost of Sales

Our cost of sales relates to sales of EXONDYS 51 following its commercial launch in the U.S. Prior to receiving regulatory approval for EXONDYS 51 from the FDA in September 2016, we expensed such manufacturing and material costs as research and development expenses. For EXONDYS 51 sold during the three months ended March 31, 2017, the majority of related manufacturing costs incurred had previously been expensed as research and development expenses, as such costs were incurred prior to the FDA approval of EXONDYS 51. Therefore, the cost of sales presented in the unaudited condensed consolidated statements of operations and comprehensive loss only included the cost of packaging and labeling for commercial sales as well as amortization of an in-licensed right. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the cost to produce the EXONDYS 51 sold would have been approximately $1.0 million for the three months ended March 31, 2017.

Research and Development Expenses

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, up-front fees and milestones paid to third parties in connection with technologies which have not reached technological feasibility and do not have an alternative future use, 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 candidates, enter or proceed through later stage 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 have started conducting Part II of a Phase 1/2a clinical trial for an exon 53-skipping product candidate in the EU. We have completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. We have initiated a placebo-controlled study with product candidates designed to skip exons 45 and 53 in the U.S. and the EU. The remainder of our research and development programs are in various stages of research and pre-clinical development. However, our research and development efforts may not result in any 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 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 revenue from the commercialization of any product candidate. The time frame for development of any product candidate, associated development costs and the probability of regulatory and commercial success vary widely.

Our research and development programs span various disease targets. The lengthy process of securing FDA approvals for new drugs requires substantial resources. Accordingly, we cannot currently estimate, with any degree of certainty, the amount of time or money that we will be required to expend in the future on our product candidates prior to their regulatory approval, if such approval is ever granted.

Research and development expenses represent a substantial percentage of our total operating expenses. 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 are not tracked on a project-by-project basis, 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 March 31,			
	2017	2016	Change	Change
	(in thousands)		$	%
EXONDYS 51	$ 8,930	$ 20,012	$ (11,082)	(55)%
Exon 53	3,422	1,893	1,529	81%
Exon 45	3,082	1,432	1,650	115%
Other projects	1,447	527	920	175%
Internal research and development expenses	12,238	14,962	(2,724)	(18)%
Total research and development expenses	$ 29,119	$ 38,826	$ (9,707)	(25)%

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

	For the Three Months Ended March 31,			
	2017	2016	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 14,362	$ 22,364	$ (8,002)	(36)%
Compensation and other personnel expenses	5,686	6,432	(746)	(12)%
Professional services	2,162	2,400	(238)	(10)%
Facility-related expenses	2,208	2,084	124	6%
Stock-based compensation	1,874	2,449	(575)	(23)%
Preclinical expenses	1,524	1,084	440	41%
Research and other	1,303	2,013	(710)	(35)%
Total research and development expenses	$ 29,119	$ 38,826	$ (9,707)	(25)%

Research and development expenses for the three months ended March 31, 2017 decreased by $9.7 million, or 25%, compared with the three months ended March 31, 2016. This was primarily driven by a decrease of $8.0 million in clinical and manufacturing expenses due to lower manufacturing expenses because of the capitalization of inventory following the approval of EXONDYS 51 by the FDA partially offset by increased patient enrollment in our ongoing clinical trials. Additionally, compensation and other personnel expenses decreased by $0.7 million and stock-based compensation by $0.6 million primarily due to a reduction in headcount in Corvallis, Oregon because of the restructuring plan implemented in March 2016.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, human resources, 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.

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

	For the Three Months Ended March 31,			
	2017	2016	Change	Change
	(in thousands)		$	%
Professional services	$ 9,741	$ 5,796	$ 3,945	68%
Compensation and other personnel expenses	8,779	7,869	910	12%
Stock-based compensation	3,838	4,241	(403)	(10)%
Other	3,858	2,970	888	30%
Total selling general and administrative expenses	$ 26,216	$ 20,876	$ 5,340	26%

Selling, general and administrative expenses for the three months ended March 31, 2017 increased by $5.3 million, or 26%, compared with the three months ended March 31, 2016. This was primarily due to increases of $3.9 million in professional services primarily due to increased legal fees and commercial initiatives and $0.9 million in compensation and other personnel expenses.

Gain from sale of Priority Review Voucher

On February 21, 2017, we entered into an agreement with Gilead Sciences, Inc. (“Gilead”) to sell our Rare Pediatric Disease Priority Review Voucher (“PRV”). We received the PRV when EXONDYS 51 was approved by the FDA for the treatment of patients with DMD amenable to exon 51 skipping. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, on March 30, 2017, we completed our sale of the PRV to a subsidiary of Gilead. Pursuant to the agreement, the subsidiary of Gilead paid us $125.0 million, which was recorded as a gain from sale of the PRV as it did not have a carrying value at the time of the sale.

Interest income (expense) and other, net

Interest income (expense) and other, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense and rental income and loss. Our cash equivalents and investments consist of commercial paper, government and

government agency debt securities, money market investments and certificates of deposit. Interest expense includes interest accrued on our senior secured term loan and our mortgage loan related to our Corvallis, Oregon property. Rental income and loss is from leasing excess space in some of our facilities.

For the three months ended March 31, 2017, interest income and other, net was approximately $0.3 million. For the three months ended March 31, 2016, interest (expense) and other, net was approximately $0.1 million. The favorable change primarily reflected increased interest income from higher balance of cash, cash equivalent and investments.

Income tax expense

Corresponding to the gain from sale of the PRV, income tax expense for the three months ended March 31, 2017 was approximately $2.0 million, primarily related to alternative minimum tax. Income tax expenses for the same period in 2016 was zero as we were in a loss position.

Liquidity and Capital Resources

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

	As of March 31, 2017	As of December 31, 2016	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 274,723	$ 122,420	$ 152,303	124%
Short-term investments	115,590	195,425	(79,835)	(41)%
Restricted cash and investments	784	11,479	(10,695)	(93)%
Total cash, cash equivalents and investments	$ 391,097	$ 329,324	$ 61,773	19%
Borrowings:				
Current portion of long-term debt	$ 11,346	$ 10,108	$ 1,238	12%
Long-term debt	2,343	6,042	(3,699)	(61)%
Total borrowings	$ 13,689	$ 16,150	$ (2,461)	(15)%
Working capital				
Current assets	$ 457,307	$ 373,476	$ 83,831	22%
Current liabilities	74,629	75,422	(793)	(1)%
Total working capital	$ 382,678	$ 298,054	$ 84,624	28%

For the period ended March 31, 2017, our principal source of liquidity was from proceeds from sale of the PRV, equity financings and product sales of EXONDYS 51. For the period ended December 31, 2016, our principal source of liquidity was from equity financings and product sales. Our principal uses of cash are research and development expenses, selling, general and administrative expenses, investments, 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:

- our ability to generate revenues from sales of EXONDYS 51 and potential future products;
- 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;
- the attainment of milestones and our obligations to make milestone payments to Summit (Oxford) Ltd, University of Western Australia and other institutions; 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 we expect to seek additional financing primarily from, but not limited to, the sale and issuance of equity, debt securities

or the licensing or sale of our technologies. We cannot provide assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Three Months Ended March 31,			
	2017	2016	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (57,888)	$ (60,688)	$ 2,800	(5)%
Investing activities	209,985	19,422	190,563	981%
Financing activities	206	(1,037)	1,243	(120)%
Increase (decrease) in cash, cash equivalents and restricted cash	$ 152,303	$ (42,303)	$ 194,606	(460)%

Operating Activities. Cash used in operating activities decreased by $2.8 million for the three months ended March 31, 2017 compared with the three months ended March 31, 2016. This was primarily due to a decrease of $20.5 million in operating loss driven by product sales for EXONDYS 51 and decreased research and development expenses partially offset by increased selling, general and administrative expenses. This was offset by a decrease of $0.7 million in non-cash adjustments and $15.4 million of unfavorable changes in operating assets and liabilities primarily related to increases in accounts receivables and inventory as we launched EXONDYS 51.

Investing Activities. The cash provided by investing activities increased by $190.6 million for the three months ended March 31, 2017 compared with the three months ended March 31, 2016. This was driven by proceeds of $125.0 million from sale of the PRV and increases of $59.0 million from maturity of available-for-sale security and $10.7 million from maturity of a restricted investment partially offset by increases of $3.3 million in purchase of property and equipment and $0.8 million in purchase of intangible assets.

Financing Activities. Cash provided by financing activities for the three months ended March 31, 2017 was approximately $0.2 million. Cash used in financing activities for the three months ended March 31, 2016 was approximately $1.0 million. The favorable change was primarily driven by an increase of $1.3 million in proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program.

Milestone Obligations

As of March 31, 2017, we were obligated to make up to $712.6 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 months ended March 31, 2017 or 2016, we did not incur any milestone obligations relating to these collaboration and license agreements.

Other Funding Commitments

As of March 31, 2017, we have several on-going clinical studies in various clinical trial stages. Our most significant clinical trial expenditures are to contract research organizations (“CROs”). The CRO contracts are generally cancellable at our option. As of March 31, 2017, we have approximately $51.0 million in cancellable future commitments based on existing CRO contracts.

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 March 31, 2017.

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 March 31, 2017, we had approximately $391.1 million of cash, cash equivalents and investments, comprised of $274.7 million of cash and cash equivalents, $115.6 million of short-term investments and $0.8 million restricted cash and investments. Our cash equivalents and short-term investments consist of commercial paper, government and government agency debt securities, corporate bonds, money market investments and certificates of deposit. The fair value of cash equivalents and short-term investments is subject to change as a result of potential changes in market interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. As of March 31, 2017, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of less than $0.1 million to our interest rate sensitive instruments.

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 March 31, 2017, 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 Chief Executive Officer and our Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of March 31, 2017, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended March 31, 2017, 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 15, 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, 2016, 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, but not limited to:

- the effectiveness of our sales, managed markets and marketing efforts;
- the effectiveness of our commercialization activities, including negotiating and entering into any additional commercial and supply contracts, scaling up manufacturing and hiring any additional personnel;
- FDA-mandated package insert requirements and the time it would take us to comply with any related FDA post-marketing requirements and commitments;
- 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;
- the adoption of EXONDYS 51 by physicians, which depends on whether physicians view it as a safe and effective treatment for patients with DMD;
- adequate reimbursement by third parties, including government payors, 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 increase awareness of the importance of genetic testing and knowing / understanding DMD mutations, and identifying and addressing procedural barriers to obtaining therapy;
- 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;
- our ability to obtain regulatory approvals to commercialize EXONDYS 51 in markets outside of the U.S.; and
- the awareness of patients with DMD of their mutation and whether the mutation is amenable to EXONDYS 51.

In addition, the process leading to a patient's first infusion of EXONDYS 51 may be slower for certain patients. For example, the time to first infusion may take longer if a patient chooses to put in an intravenous port, which eases access to the vein. As the

launch of EXONDYS 51 progresses, we expect the variation among patients to decline, leading to a faster time to infusion. However, delays in the process prior to first infusion could negatively impact the sales of EXONDYS 51.

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, in particular if they are severe, 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;
- our reputation in the marketplace may suffer; 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 payors 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. In addition, the process for adding new manufacturing capacity can be lengthy and could cause delays in our development efforts. 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 such as earthquake or fire, 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 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. While we work diligently with all contract manufacturers to maintain full compliance, we do not have direct 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 2017, 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.

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. We continue to have discussions with payors, some of which may eventually deny coverage. We may not receive approval for reimbursement of EXONDYS 51 from private insurers on a satisfactory rate or basis, in which case our business would be materially adversely affected. In addition, obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we do not receive these approvals on a timely 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.

Additionally, in the wake of government and public scrutiny of pharmaceutical pricing practices, there have been efforts at the federal and state levels to implement legislation or regulations to promote transparency in drug pricing or limit drug prices. Such initiatives are likely to continue the pressure on pharmaceutical pricing, may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs.

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 a number of factors, including current and future healthcare reforms and initiatives by government health programs and private insurers (including managed care plans) to reduce healthcare costs.

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 have aggressively pursued healthcare reform, as evidenced by the passing of the Affordable Care Act and the current debate concerning modifications to or repeal of such Act. The Affordable Care Act substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for pharmaceutical products such as increasing drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care and assessing a fee on manufacturers and importers of brand name prescription drugs reimbursed under certain government programs, including Medicare and Medicaid. 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. Modifications to or repeal of all or certain provisions of the Affordable Care Act are being actively debated as a result of the outcome of the 2016 presidential election and Republicans maintaining control of both houses of Congress, consistent with statements made by President Donald Trump and members of Congress both during the presidential campaign and continuing into 2017. We cannot predict the ultimate content, timing or effect of any changes to the Affordable Care Act or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our future business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare reform will affect our business.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. We anticipate that the U.S. Congress, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures may include:

- controls on government funded reimbursement for drugs;
- caps or mandatory discounts under certain government sponsored programs;
- controls on healthcare providers;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;

- reform of drug importation laws;
- expansion of use of managed care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person; and
- prohibition on direct-to-consumer advertising or drug marketing practices.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted, 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 net revenues and operating 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.

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 patients with DMD 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.

Continued approval for this indication is contingent upon completing various post-marketing requirements and commitments, including the requirement to conduct a randomized, controlled clinical trial to verify the drug's clinical benefit. These post-approval requirements and commitments may not be feasible and/or could impose significant burdens and costs on us; could negatively impact our development, manufacturing and supply of EXONDYS 51; and could negatively impact our financial results. Failure to meet post-approval commitments and requirements, including completion of enrollment and obtaining positive safety and efficacy data from our confirmatory studies for EXONDYS 51, would lead to negative regulatory action from the FDA and/or 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. Drug product manufacturers are required to continuously monitor and report adverse events from clinical trials and commercial use of the product. If we or a regulatory agency discover previously unknown adverse events or events of unanticipated severity or frequency, a regulatory agency may require labeling changes implementation of risk evaluation and mitigation strategy (REMS) program. If we or a regulatory agency discover previously unknown problems with a product, such as problems with a facility where the API or drug product is manufactured or tested, a regulatory agency may impose restrictions on that product and/or the manufacturer, including removal of specific product lots from the market, 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;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw or alter the conditions of our marketing approval;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- suspend any ongoing clinical trials;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- refuse to approve pending applications or supplements to applications submitted by us;

- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or require us to initiate a product recall; or
- refuse to allow us to enter into supply contracts, including government contracts.

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.

We are not permitted to market or sell EXONDYS 51 in the EU or in any other foreign countries on a commercial basis until we receive the requisite approval from such country's regulatory authorities. In order to market any product in a foreign country, we must comply with numerous and varying regulatory requirements for approval in those countries regarding demonstration of evidence of the product's safety and efficacy and governing, among other things, labeling, distribution, advertising, and promotion, as well as pricing and reimbursement of the product. Approval procedures vary among countries, and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ significantly from that required to obtain approval in the U.S. In particular, in many foreign countries, it is required that a product receives pricing and reimbursement approval before the product can be distributed commercially. This can result in substantial delays, and the price that is ultimately approved in some countries may be lower than the price for which we expect to offer EXONDYS 51.

Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the approval process in others. Failure to obtain marketing approval in other countries or any delay or setback in obtaining such approval would impair our ability to develop foreign markets for eteplirsen and could adversely affect our business and financial condition. Any such complications may reduce our target market and delay or limit the full commercial potential of eteplirsen. Many foreign countries are undertaking cost-containment measures that could affect pricing or reimbursement of eteplirsen.

In November 2016, we submitted an MAA for eteplirsen to the EMA. The application was validated in December 2016 and is currently under review. We believe that we submitted a robust package of clinical, dystrophin and safety data to support the review of eteplirsen. We also believe that, in contrast to the FDA approval, the clinical data will be central in evaluating the application, while dystrophin will be supportive of the drug's mechanism of action. However, obtaining approval of an MAA or any other filing for approval in a foreign country is an extensive, lengthy, expensive and uncertain process, and the regulatory authority may reject a filing or delay, limit or deny approval of eteplirsen for many reasons, including:

- we may not be able to demonstrate to the satisfaction of foreign regulatory authorities that eteplirsen is safe and effective for the treatment of patients with DMD who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping;
- the results of clinical trials may not meet the level of statistical or clinical significance required for approval by foreign regulatory authorities;
- foreign regulatory authorities may disagree with the adequacy (number, design, size, controls, conduct or implementation) of our clinical trials prior to granting approval, and we may not be able to generate the required data on a timely basis, or at all;
- regulatory authorities may not find the data from clinical trials sufficient to demonstrate that eteplirsen's clinical benefits outweigh its safety risks; or such regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct one or more additional trials;
- regulatory authorities outside the U.S. may not accept data generated at our clinical trial sites;
- regulatory authorities outside the U.S. may impose limitations or restrictions on the approved labeling of eteplirsen, thus limiting intended users or providing an additional hurdle for market acceptance of the product;
- regulatory authorities outside the U.S. may identify deficiencies in the manufacturing processes, or may require us to change our manufacturing process or specifications;
- we may not be able to validate our manufacturing process to the satisfaction of regulatory authorities outside the U.S. or demonstrate adequate cGMP compliance; or
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

If we are unable to execute effectively our sales and marketing activities outside the U.S., we may be unable to generate sufficient product revenue.

EXONDYS 51 is our first commercial product. As a result, we have only recently had to build our sales, marketing, managerial and other non-technical capabilities in the U.S. We plan to continue to build commercial infrastructure in the EU and in other key countries in order to be ready to launch eteplirsen with a relatively small specialty sales force if the product is ultimately approved in those jurisdictions. The establishment and development of our commercial infrastructure will continue to be expensive and time consuming, and we may not be able to successfully fully develop this capability in a timely manner or at all. We anticipate developing a commercial infrastructure across multiple jurisdictions, if eteplirsen is approved in such jurisdictions. Doing so will require a high degree of coordination and compliance with laws and regulations in such jurisdictions. If we are unable to effectively coordinate such activities or comply with such laws and regulations, our ability to commercialize eteplirsen in such jurisdictions will be adversely affected. Even if we are able to effectively hire a sales force and develop a marketing and sales infrastructure, our sales force may not be successful in commercializing eteplirsen or any other product candidate that we develop. If we are unable to establish adequate manufacturing, sales, marketing, supply and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable.

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;
- 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; and
- the efficacy and safety of competitive therapies.

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 to 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 EU for some of our product candidates, however, there can be no guarantee that we will be able to 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 our product candidates.

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 additional product candidates for the treatment of DMD and infectious diseases, including 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 EU 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 EU, our business and operations could be adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the U.S. and the EU 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 EU for the same indication as any of our product candidates for which we plan to file an NDA or 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 EU, as applicable.

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 used for the same orphan indication, except in circumstances where, based on the FDA's determination, 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 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 filed an MAA in November 2016 with the 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 1/2a 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 EU 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 follow-on exon strategy in DMD, our other 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 not be able to obtain an approval of EXONDYS 51 in the EU.

Our RNA-targeted antisense technologies have only been incorporated into one therapeutic commercial product and additional studies may not demonstrate safety or efficacy of our technologies 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, including our novel PPMO 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 technologies, 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.

Our pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, including those based on our PMO-based technologies, 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. For example, although the pre-clinical data for PPMO collected to date is promising, the additional data we collect, including in the clinic, may not be consistent with the pre-clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval for PPMO product candidates. 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, including for those that are based on our PMO-based technologies, 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 or withdrawn. For example, we cannot provide assurances that data from our EXONDYS 51 ongoing studies will be positive and consistent through the study periods or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our product candidates will be consistent with our interpretations.

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 could impair our ability to generate sufficient 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. or the EU, 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 marketing application approvals. The regulatory authorities could disagree with our interpretations and conclusions regarding data we provide in connection with NDA or MAA submissions for one of our product candidates, and may delay, reject or refuse to accept for review, or approve any NDA or MAA submission we make or identify additional requirements for product approval to be submitted upon completion, if ever. In addition, in the U.S., an FDA advisory committee could determine that 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 disagree with the advisory committee's recommendation and deny approval of a product candidate based on their review.
- 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 alternative approaches or endpoints for the determination of efficacy 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 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 for products intended to treat serious rare diseases, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned NDAs and MAAs 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, in the U.S., an FDA 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 approval of our NDA or result in a decision by the Company not to proceed with 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 and approvals may require substantial personnel, financial or other resources, which, as a small 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 with respect to any NDA or MAA 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 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.

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, certain federal and state healthcare laws and regulations will apply to or affect 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 payors that are false or fraudulent;
- 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 teaching hospitals 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 payor, including commercial insurers, state laws regulating interactions between pharmaceutical manufactures and health care providers, 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.

Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being used to enforce these laws and to prosecute companies and individuals who are believed to be violating them. In particular, the Healthcare Reform Act includes a number of provisions aimed at strengthening the government’s ability to pursue anti-kickback and false claims cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for

healthcare fraud enforcement activities, enhanced investigative powers, and amendments to the False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations. While it is too early to predict what effect these changes will have on our business, we anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions. For example, federal enforcement agencies recently have shown interest in pharmaceutical companies' product and patient assistance programs, including manufacturer reimbursement support services and relationships with specialty pharmacies. Some of these investigations have resulted in significant civil and criminal settlements. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business and financial condition and growth prospects.

In connection with the commercial launch of EXONDYS 51, we have initiated our compliance program and are in the process of expanding our experienced compliance team that will continue to work towards developing 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.

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 $39.2 million for the three months ended March 31, 2017. Our accumulated deficit was $1.1 billion as of March 31, 2017. 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 significant revenues from product sales and have generally incurred expenses related to research and development of our technologies 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:

- continue our launch and commercialization of EXONDYS 51 in the U.S.;
- expand the global footprint of EXONDYS 51 outside of 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 our 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 that 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, 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.

On April 24, 2017, Dr. Kaye informed our board of directors of his intention to resign as President and Chief Executive Officer upon the conclusion of his current employment term on September 20, 2017 or some other future date. We have begun a search for a Chief Executive Officer to replace Dr. Kaye. While Dr. Kaye will work with our management team, our board of directors, and other senior leaders at the Company to find a suitable candidate with the goal of ensuring a smooth and seamless transition, we cannot guarantee that the transition to a new Chief Executive Officer will be smooth, successful or would not result in a negative impact to the Company. Leadership transitions can be inherently difficult to manage and may cause uncertainty or a

disruption to our business or may increase the likelihood of turnover in other key officers and employees. If we lose the services of one or more of our senior management or key employees, or if one or more of them decides to join a competitor or otherwise to compete with us, our business could be harmed.

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 depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our technologies, product 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, product candidates and platform technology with a basis for exclusivity, we and our collaborators may not be able to develop or commercialize such product and 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 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 refusal of all claims of AZL's application, U.S. Application No. 13/550,210. BioMarin/AZL appealed the decision to the U.S. Court of Appeals for the Federal Circuit on October 12, 2016, and this appeal was docketed as Case No. 17-1078 and designated by the Court as a companion case to the exon 51 methods interference appeal (Case No. 16-1937) and the exon 53 interference appeal (Case No. 16-2262). We cannot make any assurances about the outcome of the appeals of any of these three interferences, or any subsequent appeals or rehearings. Any adverse rulings on the 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 appeals are not in our favor, then the Sarepta/UWA patents involved in the interferences, 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 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 invalidation proceedings in all of these countries, the outcome and timing of any such proceeding cannot be predicted or determined as of the date of this report.

The DMD patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third 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. As such, the patents and patent applications that we own or license and rely on for exclusivity for our product candidates may be challenged. In the United States, our patents may be challenged in an Inter Partes Review proceeding or other related proceeding. In other countries, other procedures are available for a third party to challenge the validity of our patent rights. For instance, we have rights to European Patent No. 2206781, which protects SRP-4053. This patent was opposed at the European Patent Office. We filed our response to the opponent's opposition statement and the European Patent Office issued a summons for oral proceeding. The outcome and timing of a final written decision from the European Patent Office 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 our 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, granted and in some cases are proceeding to grant. One of these divisional applications that has granted claims exon 45 skipping antisense oligonucleotides (EP 2594641) and another claims exon 53 skipping antisense oligonucleotides (EP 2602322). We opposed EP 2594641 and EP 2602322 in the Opposition Division on September 29, 2016 and December 1, 2016, respectively. Further divisional applications that have granted claim exon 51 skipping antisense oligonucleotides (EP 2284264 and EP 2801618). In addition, we filed suit on March 1, 2017 in the High Court of Justice in the United Kingdom seeking an order from the Court to revoke European patent (UK) Nos. 1619249, 2284264 and 2203173. Any of these granted patents, should we be unsuccessful in invalidating them or obtaining their revocation, or any related patents that grant, 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 follow on exon-skipping 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, Daiichi Sankyo and Nippon Shinyaku Co. Ltd. share a focus on RNA-targeted drug discovery and development. Competitors with respect to EXONDYS 51 or our product candidates include Nippon Shinyaku, Daiichi Sankyo, Wave and Shire plc; and other companies such as BioMarin (which acquired Prosensa), and PTC have also been working on DMD programs. Additionally, several companies and institutions 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, Roche, Biogen Idec, Inc., Ionis Pharmaceuticals, Inc., Alexion Pharmaceuticals, Inc., Sanofi, Eli Lilly, Alnylam, Moderna Therapeutics, Inc., Summit, Akashi, Catabasis, and Oxford University. Although BioMarin announced on May 31, 2016 its intent to discontinue clinical and regulatory development of drisapersen as well as its other clinical stage candidates, BMN 044, BMN 045 and BMN 053, then-currently in Phase 2 studies for distinct forms of DMD, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of DMD.

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 technologies obsolete or noncompetitive. Our competitors 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 44% 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 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 March 31, 2017, there were approximately 54.9 million shares of common stock outstanding and outstanding awards to purchase 6.5 million shares of common stock under various incentive stock plans. Additionally, as of March 31, 2017, there were approximately 2.1 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 1.0 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: May 4, 2017

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

Date: May 4, 2017

By: /s/ SANDESH MAHATME
Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business 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	Asset Purchase Agreement dated February 20, 2017 by and between Sarepta Therapeutics Inc. and Gilead Sciences, Inc.					X
10.2†	Offer Letter dated December 5, 2012 by and between Sarepta Therapeutics, Inc. and Shamim Ruff					X
10.3†	Offer Letter dated December 3, 2012 by and between Sarepta Therapeutics, Inc. and Alexander "Bo" Cumbo					X
10.4†	Offer Letter dated February 2, 2017 by and between Sarepta Therapeutics, Inc. and Dr. Catherine Stehman-Breen					X
31.1	Certification of the Company's 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 Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of the Company's 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 Executive Vice President, Chief Financial Officer and Chief Business 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

† Indicates management contract or compensatory plan, contract or arrangement.

* 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

ASSET PURCHASE AGREEMENT

BY AND BETWEEN

GILEAD SCIENCES, INC.

AND

SAREPTA THERAPEUTICS INC.

February 20, 2017

ASSET PURCHASE AGREEMENT

This Asset Purchase Agreement (this "***Agreement***") is made and entered into as of February 20, 2017 (the "***Effective Date***"), by and between GILEAD SCIENCES, INC., a corporation organized under the laws of the Delaware ("***Buyer***"), and SAREPTA THERAPEUTICS INC., a corporation organized under the laws of Delaware ("***Seller***"). Buyer and Seller may hereinafter be referred to individually as a "***Party***" and collectively as the "***Parties***".

RECITALS

WHEREAS, Seller is the holder of all right, title and interest in and to the Priority Review Voucher (as defined below).

WHEREAS, Seller and Buyer each (i) desire that Buyer purchase from Seller, and Seller sell, transfer and assign to Buyer, the Purchased Assets (as defined below), all on the terms set forth herein (such transaction, the "***Asset Purchase***") and (ii), in furtherance thereof, have duly authorized, approved and executed this Agreement and the other transactions contemplated by this Agreement in accordance with all applicable Legal Requirements (as defined below).

WHEREAS, Seller and Buyer desire to make certain representations, warranties, covenants and other agreements in connection with the Asset Purchase as set forth herein.

NOW, THEREFORE, in consideration of the foregoing and their mutual undertakings hereinafter set forth, and intending to be legally bound, the Parties hereto agree as follows:

ARTICLE I
DEFINITIONS

1.1 Certain Definitions. As used in this Agreement, the following terms shall have the meanings indicated below:

(a) "***Affiliate***" means any Person which, directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with a Party to this Agreement, for so long as such control exists, whether such Person is or becomes an Affiliate on or after the Effective Date. A Person shall be deemed to "control" another Person if it: (i) with respect to such other Person that is a corporation, owns, directly or indirectly, beneficially or legally, at least fifty percent (50%) or more of the outstanding voting securities or capital stock (or such lesser percentage which is the maximum allowed to be owned by such Person in a particular jurisdiction) of such other Person, or, with respect to such other Person that is not a corporation, has other comparable ownership interest; or (ii) has the power, whether pursuant to contract, ownership of securities or otherwise, to direct the management and policies of such other Person.

(b) "***Business Day***" means a day (i) other than Saturday or Sunday and (ii) on which commercial banks are open for business in New York, New York.

(c) "***Confidential Information***" means (i) any and all confidential and proprietary information, including but not limited to, data, results, conclusions, know-how, experience, financial information, plans and forecasts, that may be delivered, made available, disclosed or communicated by a Party or its Affiliates or their respective Representatives to the other Party or its Affiliates or their respective Representatives, related to the subject matter hereof or otherwise in connection with this Agreement and (ii) the terms, conditions and existence of this Agreement. "Confidential Information"

will not include information that (A) at the time of disclosure, is generally available to the public, (B) after disclosure hereunder, becomes generally available to the public, except as a result of a breach of this Agreement by the recipient of such information, (C) becomes available to the recipient of such information from a Third Party that is not legally or contractually prohibited by the disclosing Party from disclosing such Confidential Information; or (D) was developed by or for the recipient of such information without the use of or reference to any of the Confidential Information of the disclosing Party or its Affiliates, as evidenced by the recipient's contemporaneous written records. Notwithstanding anything herein to the contrary, all Confidential Information included within the Purchased Assets shall constitute Confidential Information of the Buyer from and after the Closing Date.

(d) "***Contract***" means any written or oral legally binding contract, agreement, instrument, commitment or undertaking (including leases, licenses, mortgages, notes, guarantees, sublicenses, subcontracts and purchase orders).

(e) "***Encumbrance***" means any lien, pledge, charge, mortgage, easement, encroachment, imperfection of title, title exception, title defect, right of possession, lease, security interest, encumbrance, adverse claim, interference or restriction on use or transfer.

(f) "***FDA***" means the United States Food and Drug Administration.

(g) "***FDCA***" means the United States Federal Food, Drug, and Cosmetic Act, as amended.

(h) "***Governmental Entity***" means any supranational, national, state, municipal, local or foreign government, any court, tribunal, arbitrator, administrative agency, commission or other governmental official, authority or instrumentality, in each case whether domestic or foreign, any stock exchange or similar self-regulatory organization or any quasi-governmental or private body exercising any regulatory, taxing or other governmental or quasi-governmental authority.

(i) "***HSR Act***" means the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and the rules and regulations promulgated thereunder.

(j) "***Legal Requirements***" means any federal, state, foreign, local, municipal or other law, statute, constitution, principle of common law, resolution, ordinance, code, rule, regulation, ruling or requirement issued, enacted, adopted, promulgated, implemented or otherwise put into effect by or under the authority of any Governmental Entity and any Orders applicable to a Party or to any of its assets, properties or businesses. Legal Requirements shall include, with respect to Seller, any responsibilities, requirements, parameters and conditions relating to the Priority Review Voucher set forth in the NDA 206488 approval letter from the Department of Health and Human Services to Seller, Reference ID 3987286, regarding approval of the Subject NDA (as defined below).

(k) "***Liabilities***" means all debts, liabilities and obligations, whether presently in existence or arising hereafter, accrued or fixed, absolute or contingent, matured or unmatured, determined or determinable, asserted or unasserted, known or unknown, including those arising under any law, action or governmental order and those arising under any Contract.

(l) "***Order***" means any order, decree, edict, injunction, writ, award or judgment of any Governmental Entity.

(m) "***Person***" means any natural person, company, corporation, limited liability company, general partnership, limited partnership, trust, proprietorship, joint venture, business organization or Governmental Entity.

(n) "***Priority Review***" means a priority review of and action upon a human drug application by the FDA not later than six (6) months after the filing of such application to the FDA, as defined in the FDCA (21 U.S.C. 360ff(a)(1)).

(o) "***Priority Review Voucher***" means the priority review voucher issued by the United States Secretary of Health and Human Services, Food and Drug Administration, to Seller, as evidenced in the U.S. Federal Register by the notice set forth at https://www.federalregister.gov/documents/2016/10/17/2016-24947/issuance-of-priority-review-voucher-rare-pediatric-disease-product (81 Federal Register 8171511) for tracking number PRV NDA 206488, as the sponsor of a rare pediatric disease product application, that entitles the holder of such voucher to Priority Review of a single human drug application submitted under Section 505(b)(l) of the FDCA or a single biologic application submitted under Section 351 of the Public Health Service Act, as further defined in the FDCA (21 U.S.C. 360ff(a)(2)).

(p) "***Proceeding***" means any action, arbitration, audit, hearing, investigation, proceeding, litigation or suit (whether civil, criminal, administrative, judicial or investigative, whether formal or informal, whether public or private) commenced, brought, conducted or heard by or before, or otherwise involving, any Governmental Entity or arbitrator.

(q) "***Purchased Assets***" means (i) the Priority Review Voucher, and (ii) any and all rights, benefits and entitlements afforded to the holder of the Priority Review Voucher.

(r) "***Regulatory Change***" means any (i) new Legal Requirement, amendment or supplement to any then-existing Legal Requirement enacted, adopted or approved by any Governmental Entity in the United States, or (ii) term or condition imposed by the FDA on the Priority Review Voucher that is not generally imposed on priority review vouchers under the FDCA as of the Effective Date, that in either case (i) or (ii) has been enacted, adopted, approved or imposed between the Effective Date and the Closing Date and adversely impacts the manner in which Buyer may use, receive, hold or otherwise exploit the Priority Review Voucher.

(s) "***Representative***" means, with respect to a particular Person, any director, officer, manager, employee, agent, consultant, advisor, accountant, financial advisor, legal counsel or other representative of that Person.

(t) "***Subject NDA***" means NDA 206488 for Exondys 51 (eteplirsen) Injection, 50mg per mL.

(u) "***Third Party***" means any Person other than a Party and such Party's Affiliates.

Other capitalized terms defined elsewhere in this Agreement and not defined in this Section 1.1 shall have the meanings assigned to such terms in this Agreement.

ARTICLE II
PURCHASE AND SALE

2.1 Purchase and Sale; No Assumed Liabilities.

(a) Upon the terms and subject to the conditions of this Agreement, Buyer agrees to purchase from Seller, and Seller agrees to sell, transfer, convey, assign and deliver to Buyer, at the Closing all of Seller's right, title and interest in, to and under the Purchased Assets, in each case free and clear of all Encumbrances.

(b) For the avoidance of doubt, (i) the sale, assignment, transfer and conveyance of the Purchased Assets from Seller to Buyer shall not include the transfer, conveyance or assumption of any Liabilities from Seller to Buyer, and (ii) Buyer shall not assume or be liable for any Liabilities of Seller or its Affiliates (fixed, contingent or otherwise, and whether or not accrued), including Liabilities relating to the Purchased Assets (other than such obligations as are imposed generally by applicable Legal Requirements solely on the holder of the Priority Review Voucher in respect of its use or transfer following the sale thereof pursuant to this Agreement) (such Liabilities, "***Excluded Liabilities***").

2.2 Purchase Price. The total consideration (the "***Purchase Price***") to be paid by Buyer to Seller for all of the Purchased Assets shall be One Hundred and Twenty Five Million Dollars (U.S. $125,000,000) due and payable upon the Closing Date.

2.3 Method of Payment. Payment of the Purchase Price to Seller shall be made in cash by wire transfer of immediately available funds to a bank account specified by Seller in writing to Buyer in the form of Valid Account Details no later than five (5) Business Days prior to the Closing Date. "***Valid Account Details***" means, with respect to any bank account, the valid (a) name of bank, (b) bank's address, (c) account number, (d) account name and (e) ABA/Routing number.

2.4 Tax Withholding. If any Legal Requirement requires deductions from, or that taxes be withheld on, payment of the Purchase Price, Buyer shall deduct or withhold such amounts from the Purchase Price and pay the applicable amount to the proper Governmental Entity. To the extent any such withheld amounts are subject to refund or credit, upon Seller's reasonable request Buyer shall cooperate, including completion and submission of any related documentation or filings, to assist Seller in obtaining any such refund or credit. Seller shall provide a complete and executed Form W-9 that certifies Seller's eligibility for reduced or non-withholdings no later than five (5) Business Days prior to the Closing Date.

ARTICLE III
CLOSING

3.1 Closing. The consummation of the purchase and sale transaction contemplated by this Agreement (the "***Closing***") shall be conducted telephonically or via email, facsimile transfer or other similar means of correspondence on such date to be mutually agreed upon by Buyer and Seller, which date shall be no later than the third (3rd) Business Day after all of the conditions set forth in ARTICLE VI have been satisfied or waived (other than those conditions which, by their terms, are intended to be satisfied at the Closing, but subject to satisfaction or waiver of such conditions). The date on which the Closing actually takes place is referred to in this Agreement as the "***Closing Date***".

3.2 Transactions to be Effected at Closing. At the Closing,

(a) Seller shall deliver, or cause to be delivered, to Buyer an executed Bill of Sale substantially in the form attached hereto as Exhibit A;

(b) Seller shall deliver, or cause to be delivered, to Buyer an executed certificate from a duly authorized officer of the Seller certifying as to the matters set forth in Section 6.2(c);

(c) Buyer shall deliver, or cause to be delivered, to Seller an executed certificate from a duly authorized officer of the Buyer certifying as to the matters set forth in Section 6.3(c);

(d) Seller shall deliver, or cause to be delivered, to Buyer an executed certificate of the secretary or an assistant secretary (or equivalent duly authorized officer or other representative) of Seller certifying (i) that attached thereto are true and complete copies of all resolutions adopted by the board of directors of Seller authorizing the execution, delivery and performance of this Agreement and the consummation of the transactions contemplated hereby, and that all such resolutions are in full force and effect and are all the resolutions adopted in connection with the transactions contemplated hereby, and (ii) as to the incumbency of each person executing this Agreement and any other document delivered in connection herewith on behalf of Seller and that the signature of each such person on this Agreement and such other document is such person's genuine signature;

(e) Buyer shall pay the Purchase Price to Seller by wire transfer of immediately available funds to an account or accounts designated in writing by Seller to Buyer in the form of Valid Account Details, such designation to occur at least five (5) Business Days prior to the Closing Date;

(f) Seller shall submit to the FDA (in the form of a submission to the Subject NDA) and deliver to Buyer a letter addressed to Buyer, substantially in the form set forth on Exhibit B hereto and duly executed by Seller, acknowledging the transfer of the Priority Review Voucher from Seller to Buyer, in accordance with applicable Legal Requirements; and

(g) Buyer shall submit to the FDA (in the form of a submission to the Subject NDA) and deliver to Seller a letter addressed to Seller, substantially in the form set forth on Exhibit C hereto and duly executed by Buyer, acknowledging the transfer of the Priority Review Voucher from Seller to Buyer, in accordance with applicable Legal Requirements.

3.3 Title Passage. Upon the Closing, all of the right, title and interest of Seller in and to the Purchased Assets shall pass to Buyer.

ARTICLE IV
REPRESENTATIONS AND WARRANTIES OF SELLER

Seller represents and warrants to Buyer, as of the Effective Date and the Closing Date, as follows:

4.1 Organization, Standing and Power. Seller is a corporation duly organized and validly existing under the laws of the State of Delaware. Seller has the corporate power and authority to own, operate and lease its properties and to carry on its business as presently conducted and is duly qualified or licensed to do business and is in good standing in each jurisdiction where the character of its properties owned or leased or the nature of its activities make such qualification or licensing necessary, except where the failure to be so qualified or licensed would not, individually or in the aggregate, reasonably be expected to adversely affect any of the Purchased Assets or Seller's ability to consummate the transactions contemplated by this Agreement. Seller is not in violation of its certificate of incorporation or bylaws, in each case as amended to date.

4.2 Due Authority. Seller has the requisite corporate power and authority to enter into and perform its obligations under this Agreement. The execution, delivery and performance of this Agreement, and the consummation of the Asset Purchase, have been duly and validly approved and authorized by all necessary corporate action on the part of Seller, and this Agreement has been duly executed and delivered by Seller. This Agreement, upon execution by the Parties, will constitute a valid and binding obligation of Seller enforceable against Seller in accordance with its terms, subject only to the effect, if any, of (a) applicable bankruptcy and other similar laws affecting the rights of creditors generally and (b) rules of law governing specific performance, injunctive relief and other equitable remedies. The approval of Seller's stockholders is not required for the execution, delivery and performance of this Agreement, and the consummation of the Asset Purchase.

4.3 Noncontravention. The execution and delivery by Seller of this Agreement does not, and the consummation of the transactions contemplated hereby, including the transfer of title to, ownership in, and possession of the Purchased Assets, will not, (a) result in the creation of any Encumbrance on any of the Purchased Assets or (b) conflict with, or result in any violation of or default under (with or without notice or lapse of time, or both), or give rise to a right of termination, cancellation or acceleration of any obligation or loss of any benefit under, or require any consent, approval or waiver from any Person pursuant to, (i) any provision of the certificate of incorporation or bylaws of Seller, in each case as amended to date, (ii) any Contract to which Seller is a party or by which it is bound which involves or affects in any way any of the Purchased Assets or (iii) except as may be required to comply with the HSR Act, any Legal Requirements applicable to Seller or any of the Purchased Assets.

4.4 No Consents. Except for the letters referenced in Sections 3.2(d) and 3.2(e) and the filing of a Premerger Notification and Report Form under the HSR Act, no filing, authorization, consent, approval, permit, order, registration or declaration, governmental or otherwise, is necessary to enable or authorize Seller to enter into, and to perform its obligations under, this Agreement.

4.5 Title to Purchased Assets. Seller is the sole and exclusive owner of the Purchased Assets and owns and at the Closing will transfer to Buyer good and transferable title to the Purchased Assets free and clear of any Encumbrances. Seller has performed all actions necessary to perfect its ownership of, and its ability to transfer, the Purchased Assets pursuant to this Agreement. Seller has provided to Buyer a true, correct and complete copy of the Priority Review Voucher.

4.6 Contracts. Except for this Agreement, there is no Contract to which Seller or any Affiliate of Seller is a party that involves or affects the ownership of, licensing of, title to, or use of any of the Purchased Assets.

4.7 Compliance With Legal Requirements. Seller and its Affiliates are, and at all times have been, in full compliance with each Legal Requirement that is or was applicable to (a) Seller's and its Affiliates' conduct, acts, or omissions with respect to any of the Purchased Assets or (b) any of the Purchased Assets. Seller and its Affiliates have not received any notice or other communication (whether oral or written) from any Person regarding any actual, alleged, possible or potential violation of, or failure to comply with, any such Legal Requirement.

4.8 Legal Proceedings. There is no pending, or to Seller's knowledge, threatened Proceeding that involves or affects (or may involve or affect) the ownership of, licensing of, title to, or use of any of the Purchased Assets. None of the Purchased Assets are subject to any Order of any Governmental Entity or arbitrator.

4.9 Governmental Authorizations. Seller is not required to hold any license, registration, or permit issued by any Governmental Entity to own, use or transfer the Purchased Assets, other than such licenses, registrations or permits that have already been obtained.

4.10 Solvency. Seller is not entering into this Agreement with the actual intent to hinder, delay, or defraud any creditor of Seller. The remaining assets of Seller after the Closing will not be unreasonably small in relation to the business in which Seller will engage after the Closing. Upon and immediately following the Closing Date, after giving effect to all of the transactions contemplated by and in this Agreement (including the payment of the Purchase Price), Seller will not be insolvent and will have sufficient capital to continue in business and pay its debts as they become due.

4.11 Revocation; Regulatory Change. The Priority Review Voucher has not been terminated, cancelled or revoked and Seller, to its knowledge, has not done or omitted to do any act (including any act which could reasonably be expected to result in the FDA invoking its policy respecting "Fraud, Untrue Statements of Material Facts, Bribery and Illegal Gratuities" set forth in 56 Fed. Reg. 46191 (September 10, 1991)), which act or omission would reasonably be expected to result in the termination, cancellation or revocation of the Priority Review Voucher. Since the date that the Priority Review Voucher was issued, to the knowledge of the Seller, there has not occurred any Regulatory Change (which definition, for the purpose of this Section 4.11, shall mean any change of the kind described in clause (ii) of such definition and be measured from the date of the issuance of the Priority Review Voucher).

4.12 Marketed Product. Seller has initiated marketing in the United States of the rare pediatric disease product for which the Priority Review Voucher was awarded within the 365-day period beginning on the date of the FDA approval of such rare pediatric disease product. The rare pediatric disease product application for which the Priority Review Voucher was awarded was not submitted by Seller to the FDA prior to October 7, 2012.

4.13 Document Disclosure. Attached as Schedule 4.13 is a true, correct and complete list of all documents for which true, correct and complete copies have been made available to Buyer as of the close of business on the last Business Day immediately preceding the Closing Date, which list includes any and all communications between Seller or its Affiliates, on the one hand, and the FDA, on the other hand, with respect to the Purchased Assets.

4.14 Intent to Use. Neither Seller nor any of its Affiliates has filed or submitted to the FDA a notification of intent to use the Priority Review Voucher, as described in 21 USC 360ff(b)(4)(A).

4.15 No Broker. Except for Credit Suisse Securities (USA) LLC, the fees and expenses of which shall be paid by Seller, there is no investment banker, broker, finder or other intermediary which has been authorized to act on behalf of Seller who might be entitled to any fee or commission in connection with the transactions contemplated by this Agreement.

ARTICLE V
REPRESENTATIONS AND WARRANTIES OF BUYER

Buyer represents and warrants to Seller, as of the Effective Date and the Closing Date, as follows:

5.1 Organization, Standing and Power. Buyer is a corporation duly organized and validly existing under the laws of the State of Delaware. Buyer has the corporate power and authority to own, operate and lease its properties and to carry on its business as presently conducted and is duly qualified or licensed to do business and is in good standing in each jurisdiction where the character of its properties owned or leased or the nature of its activities make such qualification or licensing necessary, except where the failure to be so qualified or licensed would not, individually or in the aggregate, reasonably be expected to adversely affect Buyer's ability to consummate the transactions contemplated by this Agreement. Buyer is not in violation of its certificate of incorporation or bylaws, in each case as amended to date.

5.2 Authority. Buyer has the requisite corporate power and authority to enter into and perform its obligations under this Agreement. The execution, delivery and performance of this Agreement, and the consummation of the Asset Purchase, have been duly and validly approved and authorized by all necessary corporate action on the part of Buyer, and this Agreement has been duly executed and delivered by Buyer. This Agreement, upon execution by the Parties, will constitute a valid and binding obligation of Buyer enforceable against Buyer in accordance with its terms, subject only to the effect, if any, of (a) applicable bankruptcy and other similar laws affecting the rights of creditors generally and (b) rules of law governing specific performance, injunctive relief and other equitable remedies.

5.3 Noncontravention. The execution and delivery by Buyer of this Agreement does not, and the consummation of the transactions contemplated hereby will not, conflict with, or result in any violation of or default under (with or without notice or lapse of time, or both), or give rise to a right of termination, cancellation or acceleration of any obligation or loss of any benefit under, or require any consent, approval or waiver from any Person pursuant to, (a) any provision of the certificate of incorporation or bylaws of Buyer, in each case as amended to date, (b) any Contract to which Buyer is a party or by which it is bound which involves or affects in any way the Asset Purchase or (c) except as may be required to comply with the HSR Act, any Legal Requirements applicable to Buyer.

5.4 No Consents. Except for the letters referenced in Sections 3.2(d) and 3.2(e) and the filing of a Premerger Notification and Report Form under the HSR Act, no filing, authorization, consent, approval, permit, order, registration or declaration, governmental or otherwise, is necessary to enable or authorize Buyer to enter into, and to perform its obligations under, this Agreement.

ARTICLE VI
CONDITIONS TO CLOSING

6.1 Conditions Precedent of Buyer and Seller. Each Party's obligations to consummate the transactions contemplated by this Agreement are subject to the satisfaction or waiver, at or prior to the Closing Date, of each of the following conditions precedent:

(a) HSR Act. The applicable waiting period under the HSR Act relating to the transactions contemplated by this Agreement shall have expired or been terminated.

(b) No Injunctions or Restraints. No temporary restraining order, preliminary or permanent injunction or other material legal restraint or prohibition issued or promulgated by a Governmental Entity preventing the consummation of the transactions contemplated by this Agreement shall be in effect, and there shall not be any applicable Legal Requirement that makes consummation of the transactions contemplated by this Agreement illegal.

(c) No Governmental Litigation. There shall not be any Proceeding commenced or pending by a Governmental Entity seeking to prohibit, limit, delay, or otherwise restrain the consummation of this Agreement and/or the transactions contemplated hereby.

6.2 Buyer's Conditions Precedent. The obligations of Buyer to consummate the transactions contemplated by this Agreement are subject to the satisfaction or waiver, at or prior to the Closing Date, of each of the following conditions precedent:

(a) Accuracy of Representations. Each of the representations and warranties made by Seller in this Agreement (other than the representations and warranties made by Seller in Sections 4.2, 4.5, 4.11, 4.12, 4.14, and 4.15) shall be true and correct in all respects at and as of the Closing Date (or, if made as of a specified period or date, as of such period or date), provided that any such failure of such representations and warranties to be true and correct shall be disregarded if it would not, individually or in the aggregate, reasonably be expected to delay, restrict, limit, preclude or otherwise negatively impact in a material manner the transfer and/or use of the Purchased Assets to or by Buyer. Each of the representations and warranties made by Seller in Sections 4.2, 4.5, 4.11, 4.12 , 4.14, and 4.15 shall be true and correct in all respects at and as of the Closing Date (or, in each case, if made as of a specified period or date, as of such period or date).

(b) Performance of Covenants. All of the covenants and obligations that Seller is required to comply with or to perform hereunder at or prior to the Closing Date shall have been complied with and performed in all material respects.

(c) Closing Certificate. Seller shall have delivered to Buyer a certificate, dated the Closing Date and duly executed by Seller, certifying that the conditions set forth in Sections 6.2(a) and 6.2(b) have been satisfied.

(d) No Regulatory Change. There shall not have occurred and remain in effect any Regulatory Change

(e) Lender Consent. The Consent and Second Amendment to Credit and Security Agreement dated as of February 20, 2017 by and Among Seller and Midcap Financial Trust, and the consent thereunder, shall not have been revoked and shall be in full force and effect.

6.3 Seller's Conditions Precedent. The obligations of Seller to consummate the transactions contemplated by this Agreement are subject to the satisfaction or waiver, at or prior to the Closing Date, of each of the following conditions precedent:

(a) Accuracy of Representations. Each of the representations and warranties made by Buyer in this Agreement shall be true and correct in all material respects at and as of the Closing Date (or, if made as of a specified period or date, as of such period or date), except to the extent that such representations and warranties are qualified by the term "material", or words of similar import, in which case such representations and warranties (as so written, including the terms "material", or words of similar import) shall be true and correct in all respects at and as of the Closing Date (or, if made as of a specified period or date, as of such period or date).

(b) Performance of Covenants. All of the covenants and obligations that Buyer is required to comply with or to perform hereunder at or prior to the Closing Date shall have been complied with and performed in all material respects.

(c) Closing Certificate. Buyer shall have delivered to Seller a certificate, dated the Closing Date and duly executed by Buyer, certifying that the conditions set forth in Sections 6.3(a) and 6.3(b) have been satisfied.

ARTICLE VII
PRE-CLOSING COVENANTS AND AGREEMENTS

7.1 The Parties shall use their commercially reasonable efforts to take, or cause to be taken, all actions and to do, or cause to be done, all things necessary or desirable under applicable Legal Requirements to consummate the transactions contemplated by this Agreement. Without limiting the foregoing, Seller and Buyer shall file, or shall cause their ultimate parent entities as defined in the HSR Act to file, as soon as practicable (but not later than ten Business Days) after the Effective Date, any notifications required under the HSR Act, and shall respond as promptly as practicable to all inquiries or requests received from the Federal Trade Commission, the Antitrust Division of the Department of Justice or any other Governmental Entity for additional information or documentation. In connection therewith, the Parties shall, or shall cause their respective Affiliates to, (a) furnish to the other Party such necessary information and reasonable assistance as the other Party may reasonably request in connection with its preparation of any filing or submission that is necessary under the HSR Act, and (b) keep the other Party reasonably apprised of the status of any communications with, and any inquiries or requests for additional information from the applicable Governmental Entity.

7.2 Subject to applicable confidentiality restrictions or restrictions required by applicable Legal Requirements, each Party will notify the other promptly upon the receipt of (a) any comments or questions from any Governmental Entity in connection with any filings made pursuant to Section 7.1 or the transactions contemplated by this Agreement and (b) any request by any Governmental Entity for information or documents relating to an investigation of the transactions contemplated by this Agreement. Without limiting the generality of the foregoing, each Party shall provide to the other (or the other's respective advisors) upon request copies of all correspondence between such Party and any Governmental Entity relating to the transactions contemplated by this Agreement. The Parties may, as they deem advisable and necessary, designate any competitively sensitive materials provided to the other under this Section 7.2 as "outside counsel only." Such materials and the information contained therein shall be given only to outside counsel of the recipient and will not be disclosed by such outside counsel to employees, officers, or directors of the recipient without the advance written consent of the Party providing such materials. In addition, to the extent reasonably practicable, all discussions, telephone calls, and meetings with a Governmental Entity regarding the transactions contemplated by this Agreement shall include representatives of both Parties. Subject to applicable Legal Requirements, the Parties will consult and cooperate with each other in connection with any analyses, appearances, presentations, memoranda, briefs, arguments, and proposals made or submitted to any Governmental Entity regarding the transactions contemplated by this Agreement by or on behalf of any Party.

7.3 Notwithstanding the foregoing, nothing in this Agreement shall require, or be construed to require, the Parties or any of their respective Affiliates to offer or agree to (a) (i) sell, hold, hold separate, divest, license, discontinue or limit, before or after the Closing Date, any assets, businesses, equity holdings, intellectual property, or other interests or (ii) any conditions relating to, or changes or restrictions in, the operations of any such assets, businesses, equity holdings, intellectual property or interests (including but not limited to any requirements to enter into new contracts or modify or terminate existing contracts) or (b) any material modification or waiver of the terms and conditions of this Agreement.

7.4 Until the earlier of the Closing or the termination of this Agreement, Seller shall provide Buyer with prompt written notification of the occurrence of any Regulatory Change (which definition, for purposes of this Section 7.4, shall mean any change of the kind described in clause (ii) of such definition).

7.5 Until the earlier of the Closing or the termination of this Agreement, Seller shall use commercially reasonable efforts to maintain the Priority Review Voucher in full force and effect and shall not (a) sell, assign, transfer or convey the Priority Review Voucher to any Person other than Buyer or enter into any Contract with respect thereto or (b) encumber or otherwise grant or allow to exist any Encumbrance on the Priority Review Voucher (other than pursuant to this Agreement).

ARTICLE VIII
INDEMNIFICATION

8.1 Indemnification.

(a) Indemnification by Seller. From and after the Closing, Seller will indemnify, defend and hold Buyer and its Affiliates, and their respective directors, officers, employees and agents harmless for, from and against any and all Liabilities, losses, damages, claims, costs and expenses (including reasonable attorneys' fees) (collectively, "***Damages***") arising out of any third party claims ("***Claims***") resulting from (i) any breach of Seller's representations, warranties, covenants or obligations under this Agreement or any certificate delivered by Seller hereunder, (ii) Seller's grossly negligent and/or wrongful acts, omissions or misrepresentations, regardless of the form of action, in connection with this Agreement, and/or (iii) any Excluded Liabilities.

(b) Indemnification by Buyer. From and after the Closing, Buyer will indemnify, defend and hold Seller and its Affiliates, and their respective directors, officers, employees and agents harmless for, from and against any and all Damages arising out of any Claims resulting from (i) any breach of Buyer's representations, warranties, covenants or obligations under this Agreement or any certificate delivered by Buyer hereunder, (ii) Buyer's grossly negligent and/or wrongful acts, omissions or misrepresentations, regardless of the form of action, in connection with this Agreement, and/or (iii) Buyer's, its Affiliates', or any subsequent transferee's use or ownership of the Purchased Assets.

8.2 Indemnification Procedures.

(a) A Person entitled to indemnification pursuant to Section 8.1 will hereinafter be referred to as an *"**Indemnitee**."* A Party obligated to indemnify an Indemnitee hereunder will hereinafter be referred to as an *"**Indemnitor**."* Indemnitee shall inform Indemnitor of any Claim as soon as reasonably practicable after the Claim arises, it being understood and agreed that the failure to give such notice will not relieve the Indemnitor of its indemnification obligation under this Agreement except and only to the extent that such Indemnitor is actually and materially prejudiced as a result of such failure to give notice.

(b) If the Indemnitor has acknowledged in writing to the Indemnitee the Indemnitor's responsibility for defending such Claim and such Claim is not a class action or criminal matter, the Indemnitor shall have the right to defend, at its sole cost and expense, such Claim by all appropriate proceedings, which proceedings shall be prosecuted diligently by the Indemnitor to a final conclusion or settled at the discretion of the Indemnitor; provided, however, that the Indemnitor may not enter into any compromise or settlement unless (i) such compromise or settlement includes as an unconditional term thereof, the giving by each claimant or plaintiff to the Indemnitee of a release from all liability in respect of such Claim; and (ii) the Indemnitee consents to such compromise or settlement, which consent shall not be unreasonably withheld or delayed unless such compromise or settlement

involves (A) any admission of legal wrongdoing by the Indemnitee, (B) any payment by the Indemnitee that is not indemnified hereunder or (C) the imposition of any equitable relief against the Indemnitee, in which case ((A) – (C)) the Indemnitee may withhold its consent in its sole discretion. If a good faith and diligent defense is not being or ceases to be materially conducted by the Indemnitor, the Indemnitee shall have the right, at the expense of the Indemnitor, upon at least ten (10) Business Days' prior written notice to the Indemnitor of its intent to do so, to undertake the defense of such Claim for the account of the Indemnitor (with counsel reasonably selected by the Indemnitee and approved by the Indemnitor, such approval not to be unreasonably withheld or delayed). If the Indemnitee is defending such Claim, the Indemnitee shall keep the Indemnitor apprised of all material developments with respect to such Claim and promptly provide the Indemnitor with copies of all correspondence and documents exchanged by the Indemnitee and the opposing party(ies) to such litigation. If the Indemnitor has elected to defend such Claim or if the Indemnitor has otherwise acknowledged in writing its responsibility for indemnifying a Claim, the Indemnitee may not compromise or settle such litigation without the prior written consent of the Indemnitor, such consent not to be unreasonably withheld or delayed.

(c) The Indemnitee may participate in, but not control, any defense or settlement of any Claim controlled by the Indemnitor pursuant to this Section 8.2 and shall bear its own costs and expenses with respect to such participation; provided, however, that the Indemnitor shall bear such costs and expenses if counsel for the Indemnitor shall have reasonably determined that such counsel may not properly represent both the Indemnitor and the Indemnitee.

ARTICLE IX
TERMINATION

9.1 Termination Prior to Closing. Notwithstanding any contrary provisions of this Agreement, the respective obligations of the Parties hereto to consummate the transactions contemplated by this Agreement may be terminated and abandoned at any time before the Closing only as follows:

(a) Upon the mutual written consent of Buyer and Seller; or

(b) By either Party, by written notice to the other Party if the Closing has not occurred on or before 11:59 p.m., Cambridge Massachusetts time, on the date that is [three (3) months] from the Effective Date; provided, however, that the right to terminate this Agreement under this Section 9.1(b) shall not be available to any Party whose material breach of any provision set forth in this Agreement has resulted in the failure of the Closing to occur on or before such date.

9.2 Effect of Termination. In the event of the termination of this Agreement as provided in Section 9.1, written notice thereof shall forthwith be given to the other Party hereto specifying the provision hereof pursuant to which such termination is made, and this Agreement shall forthwith become null and void (except for the provisions of this Section 9.2, Section 10.4, ARTICLE I and ARTICLE XI, which shall survive any such termination) and there shall be no liability on the part of Buyer or Seller except for damages resulting from any breach of this Agreement prior to termination of this Agreement by Buyer or Seller.

ARTICLE X
ADDITIONAL COVENANTS

10.1 Further Assurances.

(a) The Parties shall cooperate reasonably with each other in connection with any steps required to be taken as part of their respective obligations under this Agreement, including without limitation any notifications or filings required to be made to the FDA in connection with the transfer of the Purchased Assets, and shall (i) furnish upon request to each other such further information, (ii) execute and deliver to each other such other documents, and (iii) do such other acts and things, all as the other Party may reasonably request for the purpose of carrying out the intent of this Agreement and the transactions contemplated by this Agreement, including the use by Buyer, its Affiliates or their respective successors and assigns of the Priority Review Voucher in accordance with its terms and applicable Legal Requirements.

(b) Without limiting the foregoing, Buyer and Seller agree to cooperate and assist each other with respect to all filings or notifications to any Governmental Entity related to the transfer and assignment of the Purchased Assets.

10.2 Compliance with Legal Requirements. Seller shall at all times comply in all material respects with all Legal Requirements applicable to the Purchased Assets, including any and all Legal Requirements applicable to the use or transfer of the Priority Review Voucher. Seller shall forward to Buyer any communications or notices it or its Affiliates receive from any Governmental Entity in respect of the Purchased Assets.

10.3 Marketing. Seller will continuously market in the United States the rare pediatric disease product for which the Priority Review Voucher was awarded for the 365-day period beginning on the date of the FDA approval of such rare pediatric disease product to the extent required under applicable Legal Requirements or otherwise by any applicable Governmental Entity for the continued use of, or right to transfer, the Priority Review Voucher in the United States.

10.4 Nondisclosure.

(a) Subject to disclosures permitted or contemplated by Section 10.5, with respect to Confidential Information received, the Parties will (i) keep the Confidential Information confidential, (ii) not use any Confidential Information for any reason other than to carry out the intent and purpose of this Agreement, and (iii) not disclose any Confidential Information to any Person, except in each case as otherwise expressly permitted by this Agreement or with the prior written consent of the disclosing Party.

(b) Each Party may disclose Confidential Information only to its Representatives on a need-to-know basis.

(c) Each Party will (i) enforce the terms of this Section 10.4 as to its Representatives, (ii) take such action to the extent necessary to cause its Representatives to comply with the terms and conditions of this Section 10.4, and (iii) be responsible and liable for any breach of this Section 10.4 by it or its Representatives.

(d) If a Party becomes compelled by a court or is requested by a Governmental Entity to make any disclosure that is prohibited or otherwise constrained by this Section 10.4, such Party shall provide the disclosing Party with prompt notice of such compulsion or request so that it may seek an appropriate protective order or other appropriate remedy or waive compliance with the provisions of this Section 10.4. In the absence of a protective order or other remedy, the Party subject to the requirement to disclose may disclose that portion (and only that portion) of the Confidential Information that, based upon advice of its counsel, it is legally compelled to disclose or that has been requested by such Governmental Entity; provided, however, that such Party shall use reasonable efforts to obtain reliable assurance that confidential treatment will be accorded by any Person to whom any Confidential Information is so disclosed.

10.5 Disclosures Concerning this Agreement. The Parties have mutually agreed upon the contents of a press release with respect to the execution of this Agreement, which is attached as Exhibit D hereto and shall be issued by Seller on or on the next Business Day following the Effective Date. Buyer and Seller agree not to (and to ensure that their respective Affiliates do not) issue any other press releases or public announcements concerning this Agreement without the prior written consent of the other Party (which shall not be unreasonably withheld or delayed), except as required by a Governmental Entity or applicable Legal Requirement (including the rules and regulations of any stock exchange or trading market on which a Party's (or its parent entity's) securities are traded); provided that the Party intending to disclose such information shall use reasonable efforts to provide the other Party with advance notice of such required disclosure, and an opportunity to review and comment on such proposed disclosure (which comments shall be considered in good faith by the disclosing Party). Notwithstanding the foregoing, without prior submission to or approval of the other Party, either Party may issue press releases or public announcements which incorporate information concerning this Agreement which information was included in a press release or public disclosure which was previously disclosed under the terms of this Agreement or which contains only non-material factual information regarding this Agreement. Each Party acknowledges that the other Party, or the other Party's parent entity, as a publicly traded company is legally obligated to make timely disclosures of material events relating to its business. The Parties acknowledge that either or both Parties may be obligated to file a copy of this Agreement with the United States Securities and Exchange Commission; provided that if a Party is obligated to so file a copy of this Agreement, such Party shall prepare a proposed redacted version thereof and request confidential treatment thereof, and the other Party may promptly provide its comments thereon, which comments shall be considered in good faith by the Party required to so file a copy of this Agreement.

ARTICLE XI
GENERAL PROVISIONS

11.1 Survival. Except as expressly set forth herein, the representations and warranties contained in this Agreement, and liability for the breach thereof, shall survive the Closing Date and shall remain in full force and effect for a period of three (3) years following the Closing Date; provided, however, that the representations and warranties contained in Sections 4.2, 4.5, 4.11, 4.12 , 4.14, and 4.15 hereof, and all covenants and obligations contained herein, shall, in each case, survive the Closing Date and remain in full force and effect until the expiration of the applicable statute of limitations.

11.2 Transfer Taxes and Fees . Any and all sales, excise, use, value-added and similar taxes, fees or duties assessed or incurred by reason of the sale by Seller and the purchase by Buyer of the Purchased Assets hereunder shall be shared equally between the Seller and Buyer, regardless of which Party such taxes, fees or duties are assessed against.

11.3 Notices. Any notice or other communication required or permitted to be delivered to any Party shall be in writing and shall be deemed properly delivered, given and received: (a) when delivered by hand; (b) upon such Party's receipt after being sent by registered mail, by courier or express delivery service; or (c) upon confirmation of receipt during normal business hours on a Business Day or, if received after normal business hours, on the next Business Day, after being sent by facsimile, in any case to the address or facsimile number set forth beneath the name of such Party below (or to such other address as such Party shall have specified in a written notice given to the other Party in accordance with this Section 11.3):

(i) if to Buyer, to:

Gilead Sciences, Inc.
333 Lakeside Drive
Foster City, CA 94404
United States of America
Attention: General Counsel
Facsimile: +1 650 522 5771

with a copy (which shall not constitute notice) to:

Covington & Burling LLP
One Front Street
San Francisco, California 94111
United States of America
Attention: Amy Toro and Jonas Marson
Facsimile: +1 415 591 6091

(ii) if to Seller, to:

Sarepta Therapeutics, Inc.
215 First Street, Suite 415
Cambridge, MA 02127
Attention: General Counsel

with a copy (which shall not constitute notice) to:

Cooley LLP
3175 Hanover St.
Palo Alto, CA 94304
Attention: Glen Sato
Facsimile: +1 650 849 7400

11.4 Construction.

(a) The Parties agree that any rule of construction to the effect that ambiguities are to be resolved against the drafting Party shall not be applied in the construction or interpretation of this Agreement.

(b) As used in this Agreement, the words "include" and "including," and variations thereof, shall not be deemed to be terms of limitation, but rather shall be deemed to be followed by the words "without limitation."

(c) Except as otherwise indicated, all references in this Agreement to "Articles" and "Sections" are intended to refer to Articles and Sections of this Agreement.

11.5 Counterparts. This Agreement may be executed in two or more counterparts, all of which shall be considered one and the same instrument, and shall become effective when one or more counterparts have been signed by each of the Parties hereto and delivered to the other Party hereto, it being understood that all Parties hereto need not sign the same counterpart. The exchange of a fully executed Agreement (in counterparts or otherwise) by electronic transmission or facsimile shall be sufficient to bind the Parties hereto to the terms and conditions of this Agreement.

11.6 Entire Agreement. This Agreement, including all exhibits and schedules attached hereto, sets forth the entire understanding of the Parties relating to the subject matter hereof and supersedes all prior agreements and understandings among or between the Parties relating to the subject matter hereof.

11.7 Assignment. No Party will have the right to assign this Agreement, in whole or in part, by operation of law or otherwise, without the other Party's express prior written consent. Any attempt to assign this Agreement without such consent, will be null and void. Notwithstanding the foregoing, any Party may assign this Agreement, in whole or in part, without the consent of the other Party: (a) to a Third Party that succeeds to all or substantially all of its assets or business related to this Agreement (whether by sale, merger, operation of law or otherwise); or (b) to an Affiliate of such Party. Notwithstanding the foregoing, Buyer may assign this Agreement, in whole or in part, without Seller's consent, to any purchaser, transferee, or assignee of any of the Purchased Assets. For the avoidance of doubt, no assignment made pursuant to this Section 11.7 shall relieve the assigning Party of any of its obligations under this Agreement. Subject to the foregoing, this Agreement will bind and inure to the benefit of each Party's successors and permitted assigns.

11.8 Severability. If any provision of this Agreement, or the application thereof, becomes or is declared by a court of competent jurisdiction to be illegal, void or unenforceable, the remainder of this Agreement shall continue in full force and effect and shall be interpreted so as reasonably to effect the intent of the Parties hereto. The Parties hereto shall use commercially reasonable efforts to replace such void or unenforceable provision of this Agreement with a valid and enforceable provision that shall achieve, to the extent possible, the economic, business and other purposes of such void or unenforceable provision.

11.9 Remedies Cumulative. Except as otherwise provided herein, any and all remedies herein expressly conferred upon a Party hereto shall be deemed cumulative with and not exclusive of any other remedy conferred hereby or by law or equity upon such Party, and the exercise by a Party hereto of any one remedy shall not preclude the exercise of any other remedy and nothing in this Agreement shall be deemed a waiver by any Party of any right to specific performance or injunctive relief.

11.10 Governing Law. This Agreement shall be governed by, and construed in accordance with, the laws of the State of New York, regardless of the laws that might otherwise govern under applicable principles of conflicts of law. The Parties irrevocably and unconditionally submit to the exclusive jurisdiction of the United States District Court for the Southern District of New York (or if such court does not have subject matter jurisdiction, State Court of the State of New York located in New York County) solely and specifically for the purposes of any action or proceeding arising out of or in connection with this Agreement.

11.11 Amendment; Extension; Waiver. Subject to the provisions of applicable Legal Requirements, the Parties hereto may amend this Agreement at any time pursuant to an instrument in writing signed on behalf of each of the Parties hereto. At any time, any Party hereto may, to the extent legally allowed, (a) extend the time for the performance of any of the obligations or other acts of the other Party hereto, (b) waive any inaccuracies in the representations and warranties made to such Party contained herein or (c) waive compliance with any of the agreements or conditions for the benefit of such Party contained herein. Any agreement on the part of a Party hereto to any such extension or waiver shall be valid only if set forth in an instrument in writing signed on behalf of such Party. Without limiting the generality or effect of the preceding sentence, no delay in exercising any right under this Agreement shall constitute a waiver of such right, and no waiver of any breach or default shall be deemed a waiver of any other breach or default of the same or any other provision in this Agreement.

11.12 Representation By Counsel; Interpretation. Seller and Buyer each acknowledge that it has been represented by its own legal counsel in connection with this Agreement and the transactions contemplated by this Agreement. Accordingly, any rule of law, or any legal decision that would require interpretation of any claimed ambiguities in this Agreement against the Party that drafted it, has no application and is expressly waived.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, each of Buyer and Seller has caused this Asset Purchase Agreement to be executed and delivered by their respective officers thereunto duly authorized, all as of the date first written above.

GILEAD SCIENCES, INC.

By: /s/ John F. Milligan
Name: John F. Milligan
Title: President and CEO

SAREPTA THERAPEUTICS INC.

By: /s/ Edward M. Kaye, M.D.
Name: Edward M. Kaye, M.D.
Title: President and CEO

[Signature Page to Asset Purchase Agreement]

Exhibit A

FORM OF BILL OF SALE

This Bill of Sale (this "***Bill of Sale***") is entered into as of [ ], by and between SAREPTA THERAPEUTICS INC., a corporation organized under the laws of Delaware ("***Seller***"), and GILEAD SCIENCIES, INC., a corporation organized under the laws of the State of Delaware ("***Buyer***").

Upon the terms and subject to the conditions of the Asset Purchase Agreement, dated as of February 20, 2017 (the "**Asset Purchase Agreement**"), by and between Buyer and Seller, Seller has agreed to sell, and Buyer has agreed to purchase, all right, title and interest in, to and under the Purchased Assets, including the Priority Review Voucher, in each case free and clear of all Encumbrances.

For good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, Buyer and Seller, intending to be legally bound, hereby agree as follows:

Defined Terms; Interpretation. Except as otherwise set forth herein, capitalized terms used in this Bill of Sale shall have the meanings assigned to them in the Asset Purchase Agreement. This Bill of Sale shall be interpreted in accordance with the rules of construction set forth in Section 11.4 of the Asset Purchase Agreement.

Transfer of Purchased Assets. Pursuant to the terms and subject to the conditions of the Asset Purchase Agreement, Seller hereby sells, assigns, transfers, and conveys to Buyer and its successors and its assigns, and Buyer hereby does purchase from Seller, all of Seller's right, title and interest in, to and under the Purchased Assets (including the Priority Review Voucher), in each case free and clear of all Encumbrances.

Effective Time. This Bill of Sale shall be effective as of the Closing.

Binding Effect; Amendments. This Bill of Sale shall be binding upon, inure to the benefit of, and be enforceable by, the parties hereto and their respective legal representatives, successors and permitted assigns. Neither this Bill of Sale, nor any term or provision hereof, may be amended, modified, superseded or cancelled except by an instrument in writing signed by each party hereto.

Governing Law. This Bill of Sale and any disputes arising under or related hereto shall be governed by the rules set forth in Section 11.10 of the Asset Purchase Agreement.

Counterparts. This Bill of Sale may be executed in one or more counterparts, each of which shall be deemed an original but all of which together will constitute one and the same instrument.

[*Signature Page Follows*]

IN WITNESS WHEREOF, the parties hereto have caused this Bill of Sale to be executed and delivered as of the date first written above.

GILEAD SCIENCES, INC.

By: _____
Name:
Title:

SAREPTA THERAPEUTICS INC.

By: _____
Name:
Title:

[*Signature Page to Bill of Sale*]

Exhibit B

Seller's Transfer Acknowledgment Letter

[Sarepta's Letterhead]

[Date]

Gilead Sciences, Inc.
[Buyer Contact]
[Buyer Address]

RE: NDA 206488 Exondys 51 (eteplirsen) Injection, 50mg per mL – Transfer of Rare Pediatric Disease Priority Review Voucher PRV NDA 206488 (the "***Voucher***")

Dear [Buyer Contact]:

Reference is made to the subject NDA and all related correspondence.

Please be advised that as of [Date], Gilead Sciences, Inc. ("***Buyer***") has legally accepted complete ownership of the Voucher from Sarepta Therapeutics Inc. ("***Sarepta***"). Sarepta hereby authorizes transfer of ownership of the Voucher to Buyer.

Sarepta has provided Buyer with an unredacted copy of the Exondys 51 (NDA 206488) approval letter from the Department of Health and Human Services to Sarepta (Reference ID 3987286), which includes the Voucher (the "***Approval Letter***"). Buyer agrees to use the Voucher in accordance with the terms of the Approval Letter.

Please do not hesitate to contact me should you have any questions or comments.

Sincerely,

[Sarepta Contact]

Exhibit C

Buyer's Transfer Acknowledgment Letter

[Gilead Sciences, Inc. Letterhead]

[Date]

Sarepta Therapeutics Inc.
[Seller Contact]
[Seller Address]

RE: NDA 206488 Exondys 51 (eteplirsen) Injection, 50mg per mL – Transfer of Rare Pediatric Disease Priority Review Voucher PRV NDA 206488 (the "***Voucher***")

Dear [Seller Contact]:

Reference is made to the subject NDA and related correspondence regarding PRV NDA 206488.

Please be advised that as of [Date], Gilead Sciences, Inc. ("***Buyer***") has legally accepted complete ownership of the Voucher from Sarepta Therapeutics Inc. ("***Sarepta***").

Sarepta has provided Buyer with an unredacted copy of the Exondys 51 (NDA 206488) approval letter from the Department of Health and Human Services to Sarepta (Reference ID 3987286), which includes the Voucher (the "***Approval Letter***"). Buyer will advise the U.S. Food and Drug Administration ("***FDA***") of the legal transfer of the Voucher from Sarepta to Buyer by providing a copy of this letter to the FDA, and agrees to use the Voucher in accordance with the terms of the Approval Letter.

The regulatory contact information for the Voucher is as follows:

[[Buyer] Contact]

Please do not hesitate to contact me should you have any questions or comments.

Sincerely,

[[Buyer] Contact]

Exhibit D

Press Release

Sarepta Therapeutics Agrees to Sale of Priority Review Voucher for $125M

-- Sale of PRV Provides a Significant Infusion of Non-Dilutive Capital --

CAMBRIDGE, Mass.--(BUSINESS WIRE)—February X, 2017--Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage developer of innovative RNA-targeted therapeutics, today announced it has entered into an agreement to sell its Rare Pediatric Disease Priority Review Voucher (PRV). Sarepta received the PRV when EXONDYS 51 ™ was approved by the U.S. Food and Drug Administration (FDA) for the treatment of patients with Duchenne muscular dystrophy amenable to exon 51 skipping.

The voucher was awarded by the FDA under a provision that encourages development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. With the passage of the 21st Century Cures Act, this PRV program has been extended through September 30, 2020.

As part of the agreement, Sarepta will receive an upfront payment of $125M upon the closing of the transaction, which is subject to customary closing conditions and is expected to occur following expiration of the applicable U.S. antitrust clearance requirements. Credit Suisse served as Sarepta's advisor on this transaction and conducted an extensive sales process, which included outreach to multiple pharmaceutical and biotech companies.

"Our mission at Sarepta Therapeutics is to treat more boys with Duchenne muscular dystrophy," said Edward Kaye, Sarepta's chief executive officer. "The sale of the PRV provides an important source of non-dilutive capital to support the rapid advancement of our follow on exon skipping candidates and next generation RNA targeted antisense platform."

About Sarepta Therapeutics

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. For more information, please visit us at www.sarepta.com.

About the Rare Pediatric Disease Priority Review Voucher Program

The program is intended to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. A PRV may be issued to the sponsor of a rare pediatric disease product application and would entitle the holder to priority review of a single New Drug Application or Biologics License Application, which reduces the target review time and could lead to an expedited approval. The sponsor receives the PRV upon approval of the rare pediatric disease product application and it can be sold without limitation, subject to applicable FDA requirements for filing and use.

Forward-Looking Statements

This press release contains forward-looking statements for purposes of the Private Securities Litigation Reform Act of 1995 (the "Act") and the protection of the Act's Safe Harbor for forward-looking statements. Examples of such statements include, but are not limited to, statements relating to Sarepta's commercialization and development activities, including the potential for success and timing for the progression of Sarepta's drug candidates; current regulatory requirements for approval of the purchase transaction and the viability of the priority review voucher sold; and Sarepta's capital needs. Such statements are based on management's current expectations, but actual results may differ materially due to various risks and uncertainties, including, whether regulatory requirements may change that affect the timing for payment and risk of payment under the royalty agreement with the pharmaceutical company purchaser. For further information regarding these and other risks related to Sarepta's business, investors should consult Sarepta's most recent Quarterly Report on Form 10-Q filing with the Securities and Exchange Commission. Forward-looking statements are not guarantees of future performance, and Sarepta's actual results of operations, financial condition and liquidity, and the development of the industry in which it operates, may differ materially from the forward-looking statements contained in this press release. Sarepta assumes no obligation to update its forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Source: Sarepta Therapeutics, Inc.

Media and Investors:

Sarepta Therapeutics, Inc.
Ian Estepan, 617-274-4052
iestepan@sarepta.com

Or
W2O Group
Brian Reid, 212-257-6725
breid@w2ogroup.com

Schedule 4.13

Accelerated Approval Letter from Department of Health and Human Services of the FDA to Seller dated September 19, 2016

Consent and Second Amendment to Credit and Security Agreement dated as of February 20, 2017 by and Among Seller and Midcap Financial Trust

Exhibit 10.2

November 30, 2012

Shamim Ruff
Home Address: **REDACTED**

Dear Shamim:

Congratulations on being selected as Vice President, Regulatory Affairs and Quality, at Sarepta Therapeutics (the "Company"), effective January 2, reporting to Chris Garabedian, Chief Executive Officer. Your employee orientation will begin at a mutually agreed upon time on your hire date at our Cambridge office, 245 First Street, Suite 1800, Cambridge, MA.

Your annualized starting wage will be $330,000.00 per year which will be paid semi-monthly in accordance with the Company's normal payroll procedures. As an employee, you shall be eligible to participate in the employee benefit plans and programs described in the "Summary of Major Employee Benefits" document (a copy of which is attached hereto as Exhibit A), as well as any other company benefit plans and programs available to company executives generally. Eligibility for our current benefit plans are the date of hire. You should note that the Company may modify salaries and benefits from time to time as it deems necessary.

In connection with your hire, you will receive a one-time sign-on bonus of $50,000.00 (the "Sign-on Bonus"), less any applicable withholdings, payable at the next regularly scheduled Company payroll date after your hire date. If you voluntarily resign your employment with the Company on or prior to the oneyear anniversary of your hire date, you shall be obligated to repay 100% of the Sign-On Bonus. Any such repayment must be made to the Company within sixty (60) days after your voluntary resignation from employment.

You will participate in our performance bonus program beginning in 2013 with a target rate of 30% of your base pay. The actual bonus paid could be more or less than target, depending upon the evaluation of your 2013 individual goals, as well as corporate performance for 2013 and subsequent years.

Subject to approval by the Compensation Committee of our Board of Directors after commencement of your employment you shall receive a grant of options to purchase 80,000 shares of the Company's Common Stock. Your grant will be priced in accordance with our equity incentive plan and our policies governing stock option grants.

Under our current policy, the grant date of such option will be fixed as either (i) the last trading day of the month during which a meeting of the New Employee Option Committee or Compensation Committee is held to approve such option or (ii) the effective date of an action by unanimous written consent of the Compensation Committee approving such option; provided that, the option grant shall be approved and granted to you no later than 30 days from your date of commencement of employment.

The exercise price of such option will equal the closing sales price of the Company's Common Stock as reported by The NASDAQ Global Market on the grant date. Twenty-five percent (25%) of the shares underlying the option will vest on the one-year anniversary of the date of the commencement of your employment, and 1/48th of the shares underlying the option will vest on each monthly anniversary of the date of the commencement of your employment thereafter, such that 100 percent (100%) of the shares underlying the option will be fully exercisable on the fourth annual anniversary of the date of the commencement of your employment, subject to your continued employment through any such anniversary dates. Shares shall only vest on such dates; no rights to any vesting shall be earned or accrued prior to such dates.

As a part of the employment process, we reserve the right to conduct background investigations and/or reference checks on all potential employees to the fullest extent permitted under applicable law. Your job offer, therefore, is contingent upon a clearance of such background investigation and/or reference check.

Parking

Onsite parking at the Cambridge location is available through Laz Parking (www.lazparking.com). If you elect to park in this lot, you must first fill out the appropriate paperwork and register with Laz Parking. The Company will reimburse your Laz Parking expenses up to a maximum of $130 per month, which is fifty percent (50%) of the current monthly Laz Parking fee, in accordance with the Company's expense reimbursement policy.

Employment At-Will.

If you accept our offer of employment, you will be an employee at-will, meaning that your employment is of indefinite duration and either you or the Company may terminate our employment relationship at any time for any reason, with or without cause and with or without advance notice. In the event of your resignation, we request that you give the Company at least two weeks' notice.

None of the benefits offered to you by the Company create a right to continue in employment for any particular period of time. Any statements to the contrary that may have been made to you are unauthorized and are superseded and cancelled by this offer letter. Please also remember that employment terms like your position, hours of work, work location, compensation, the stock option plan, and other employee benefits may change over the course of employment at the Company's discretion.

Severance Benefits

As of your hire date you will be entitled to enter into the Company's standard Change in Control and Severance Agreement. This agreement will outline any severance benefits you will be eligible for in the event of certain terminations of your employment and shall be provided to you for execution within five (5) days following your hire date.

Proprietary Rights Agreement.

As a condition of your employment, you are required to sign a Confidential Proprietary Rights and NonDisclosure Agreement ("Agreement"). The Agreement is enclosed to give you an opportunity to read it prior to your hire date. The Agreement must be signed on or before your hire date as a condition of employment.

We need to emphasize the importance we place on the proper treatment of all proprietary information, including that which you may have come into contact with in your prior employment. The Company is extending this offer to you based upon your general skills and abilities, and not your possession of any trade secret, confidential or proprietary information of a former employer. The Company requires that you do not obtain, keep, use for our benefit, or disclose this type of information from any prior employers to us. By accepting this offer, you will also be affirming to the Company that you are not a party to any agreement with a prior employer that would prohibit your employment with us.

Moreover, you agree that during the term of your employment, you will not engage in any other employment, occupation, consulting, or other business activity directly related to the business in which the Company is now involved or becomes involved during the term of your employment, nor will you engage in any other activities that conflict with your obligations to the Company.

Eligibility for Employment.

The United States government requires all U.S. employers to verify that employees are eligible to work in the United States. This law applies to citizens and non-citizens. Enclosed is a list of documents that are acceptable for completing the employment verification (Form 1-9) process. Please bring your documentation with you on your first day. The law requires that such documentation be provided within 3 business days of the effective date of your employment, or your employment relationship with the Company may be terminated. In addition, since the Company is a Federal contractor, please note that we participate in e-Verify (an online work authorization verification system).

Acceptance.

If you wish to accept employment with Sarepta Therapeutics, please sign this letter and return one copy to me. This offer will remain open through Friday, December 7, 2012.

This letter, along with any agreements relating to proprietary rights between you and the Company, set forth the terms of your employment with the Company and supersede any prior representations or agreements including, but not limited to, any representations made during your interviews or relocation negotiations, whether written or oral. This letter, including, but not limited to, its at-will employment provision, may not be modified or amended except by a written agreement signed by the Company CEO or President and you.

We are pleased to welcome you to Sarepta Therapeutics. If you have any questions, please give me a call at 617-444-8424.

Sincerely,

/s/ Joan Wood

Joan Wood
Vice President, Human Resources

AGREED TO AND ACCEPTED:

I accept the above written offer of employment under the terms in this letter.

Signature: /s/ Shamim Ruff Date: 5th December, 2012

Exhibit 10.3

December 3, 2012
Bo A. Cumbo
Home Address: **REDACTED**

Dear Bo:

Congratulations on being selected as Vice President, Business Development, at Sarepta Therapeutics {the "Company"), effective January 2, reporting to Chris Garabedian, Chief Executive Officer. Your employee orientation will begin at a mutually agreed upon time on your hire date at our Cambridge office, 245 First Street, Suite 1800, Cambridge, MA.

Your annualized starting wage will be $275,000.00 per year which will be paid semi-monthly in accordance with the Company's normal payroll procedures. As an employee, you may also be eligible to receive certain employee benefits offered by the Company. Eligibility for our current benefit plans are the date of hire. You should note that the Company may modify salaries and benefits from time to time as it deems necessary.

You will participate in our performance bonus program beginning in 2013 with a target rate of 30% of your base pay. The actual bonus paid could be more or less than target, depending upon the evaluation of your 2013 individual goals, as well as corporate performance for 2013 and subsequent years.

We will recommend to the Compensation Committee of our Board of Directors after commencement of your employment that you receive a grant of options to purchase 65,000 shares of the Company's Common Stock, and your grant will be subject to the approval of the Compensation Committee or its delegate. Your grant will be priced in accordance with our equity incentive plan and our policies governing stock option grants.

Under our current policy, the grant date of such option will be fixed as either (i) the last trading day of the month during which a meeting of the New Employee Option Committee or Compensation Committee is held to approve such option or (ii) the effective date of an action by unanimous written consent of the Compensation Committee approving such option. The exercise price of such option will equal the closing sales price of the Company's Common Stock as reported by The NASDAQ Global Market on the grant date. Twenty-five percent of the shares underlying the option will vest on the oneyear anniversary of the date of the commencement of your employment, and 1/48 of the shares underlying the option will vest on each monthly anniversary of the date of the commencement of your employment thereafter, such that the shares underlying the option will be fully exercisable on the fourth annual anniversary of the date of the commencement of your employment, subject to your continued employment through any such anniversary dates. Shares shall only vest on such dates; no rights to any vesting shall be earned or accrued prior to such dates.

As a part of the employment process, we reserve the right to conduct background investigations and/or reference checks on all potential employees to the fullest extent permitted under applicable law. Your job offer, therefore, is contingent upon a clearance of such background investigation and/or reference check.

Parking

Onsite parking at the Cambridge location is available through Laz Parking (www.lazparking.com). If you elect to park in this lot, you must first fill out the appropriate paperwork and register with Laz Parking. The Company will reimburse your Laz Parking expenses up to a maximum of $130 per month, which is fifty percent (50%) of the current monthly Laz Parking fee, in accordance with the Company's expense reimbursement policy.

Employment At-Will

If you accept our offer of employment, you will be an employee at-will, meaning that your employment is of indefinite duration and either you or the Company may terminate our employment relationship at any time for any reason, with or without cause and with or without advance notice. In the event of your resignation, we request that you give the Company at least two weeks' notice.

None of the benefits offered to you by the Company create a right to continue in employment for any particular period of time. Any statements to the contrary that may have been made to you are unauthorized and are superseded and cancelled by this offer letter. Please also remember that employment terms like your position, hours of work, work location, compensation, the stock option plan, and other employee benefits may change over the course of employment at the Company's discretion.

Severance Benefits

Following commencement of your employment with the company you will be eligible to enter into the company's standard Change in Control and Severance Agreement. This agreement will outline any severance benefits you may be eligible for in the event of certain terminations of your employment.

Proprietary Rights Agreement.

As a condition of your employment, you are required to sign a Confidential Proprietary Rights and Non Disclosure Agreement ("Agreement"). The Agreement is enclosed to give you an opportunity to read it prior to your hire date. The Agreement must be signed on or before your hire date as a condition of employment.

We need to emphasize the importance we place on the proper treatment of all proprietary information, including that which you may have come into contact with in your prior employment. The Company is extending this offer to you based upon your general skills and abilities, and not your possession of any trade secret, confidential or proprietary information of a former employer. The Company requires that you do not obtain, keep, use for our benefit, or disclose this type of information from any prior employers to us. By accepting this offer, you will also be affirming to the Company that you are not a party to any agreement with a prior employer that would prohibit your employment with us.

Moreover, you agree that during the term of your employment, you will not engage in any other employment, occupation, consulting, or other business activity directly related to the business in which the Company is now involved or becomes involved during the term of your employment, nor will you engage in any other activities that conflict with your obligations to the Company.

Eligibility for Employment.

The United States government requires all U.S. employers to verify that employees are eligible to work in the United States. This law applies to citizens and non-citizens. Enclosed is a list of documents that are acceptable for completing the employment verification (Form 1-9) process. Please bring your documentation with you on your first day. The law requires that such documentation be provided within 3 business days of the effective date of your employment, or your employment relationship with the Company may be terminated. In addition, since the Company is a Federal contractor, please note that we participate in e-Verify (an online work authorization verification system).

Acceptance.

If you wish to accept employment with Sarepta Therapeutics, please sign this letter and return one copy to me. This offer will remain open through Monday, December 10, 2012.

This letter, along with any agreements relating to proprietary rights between you and the Company, set forth the terms of your employment with the Company and supersede any prior representations or agreements including, but not limited to, any representations made during your interviews or relocation negotiations, whether written or oral. This letter, including, but not limited to, its at-will employment provision, may not be modified or amended except by a written agreement signed by the Company CEO or President and you.

We are pleased to welcome you to Sarepta Therapeutics. If you have any questions, please give me a call at 617-444-8424.

Sincerely,

/s/ Joan Wood

Joan Wood
Vice President, Human Resources

AGREED TO AND ACCEPTED:

I accept the above written offer of employment under the terms in this letter.

Signature: /s/ Bo Cumbo Date: December 3, 2012

Exhibit 10.4

Friday, February 3, 2017

Catherine Stehman-Breen
Home Address: **REDACTED**

Dear Catherine:

On behalf of Sarepta Therapeutics, Inc. ("Sarepta" or the "Company"), it is a great pleasure to extend you this offer of employment as Chief Medical Officer in Cambridge, MA effective on a date agreed upon following your acceptance of this offer ("Hire Date"), reporting to Edward M. Kaye, Chief Executive Officer.

Base Salary.
In this position, you will earn an annual base salary of $405,000, subject to applicable taxes and withholdings, which will be paid on a bi-weekly basis.

Future Salary Increases.
If you join Sarepta between January 1 and September 30, you will be eligible for a pro-rated merit increase for the next calendar year's Annual Compensation Review process. The Annual Compensation Review process generally takes place in the first quarter of the calendar year. Salary merit increases, if any, will be awarded at the Company's discretion on the basis of your performance, and will be pro-rated.

Annual Bonus Program.
During your employment, you will also be eligible to participate in Sarepta's annual bonus program. The target bonus opportunity for your position is 40% of your annual base salary, with the actual amount of such bonus, if any, being determined by the Company in its sole discretion, based on your performance and that of the Company against goals established by the Board. You must commence your employment by September 30 in order to be eligible for a bonus for the calendar year during which you were hired. If you join the Company between January 1 and September 30, you will be eligible for a pro-rated bonus for that calendar year. You must be employed through the date bonuses are disbursed to employees generally in order to be eligible for the bonus. Additional details regarding Sarepta's bonus program will be provided to you upon commencing employment.

New Hire Option Grant.
The Company, as an inducement for acceptance of the terms of the offer letter, plans to grant to you, subject to Compensation Committee approval, the option to purchase 100,000 shares of Company common stock pursuant to the inducement exemption contained in Nasdaq's Rule 5635(c)(4).

Under our current policy, the grant date of such option will be fixed as either (i) the last trading day of the month during which a meeting of the New Employee Option Committee or Compensation Committee is held to approve such option or (ii) the effective date of an action by unanimous written consent of the Compensation Committee approving such option. The exercise price of such option will equal the closing sales price of the Company's Common Stock as reported by The NASDAQ Global Market on the grant date. Twenty-five percent of the shares underlying the option will vest and become exercisable on the first anniversary of your hire date, and 1/48th of the shares underlying the option will vest and become exercisable on each monthly anniversary of your hire date thereafter, such that the shares underlying the option will be fully vested and exercisable on the fourth anniversary of your hire date, subject to your continued employment through each such vesting date.

Annual Equity Grant Program
You may also be eligible to be considered for the Company's annual equity grant program based on your performance. If you join the Company between January 1 and September 30 of the current calendar year, you will be eligible for a prorated annual equity grant in the calendar year that follows, with the actual amount of such equity grant, if any, being determined by the Company in its sole discretion. If you join the Company after September 30 of the current calendar year, your eligibility to participate will be postponed by one more calendar year.

Benefits.
You will be eligible to participate in the benefit plans and programs made available by the Company from time to time for employees generally, subject to plan terms and generally applicable Company policies. These currently include, but are not limited to:

- health insurance such as medical, dental and vision;
- company-paid basic life insurance, accidental death & dismemberment, and short- and long-term disability;
- paid time off such as accrued vacation, sick leave and company-paid holidays;
- 401(k) retirement savings plan; and employee stock purchase plan;

For additional details, please review the enclosed *Employees Benefits You Can Count On* document.

Relocation/Temporary Housing.
The Company will reimburse you up to $60,000 for eligible relocation expenses, subject to applicable taxes and the terms of the enclosed Relocation Agreement. Please review the Relocation Agreement for important related details and, if you agree to the terms outlined, please provide a signed copy together with your signed offer of employment letter. [You acknowledge and agree that you will not be eligible for Company reimbursement of any relocation expenses unless you accept the Relocation Agreement before incurring any such expenses.]

Background Check and Reference Check.
As a part of Sarepta's employment process, we reserve the right to conduct background checks and/or reference checks on all potential employees to the fullest extent permitted under applicable law. This offer of employment, therefore, is contingent upon your successful completion of these checks.

Parking.
As a part of Sarepta's transportation assistance program, the Company will reimburse 50% of your parking or commuting services expenses, up to $140 per month, subject to generally applicable program terms and conditions, including acceptable substantiation of eligible expenses.

Employment At-Will.
This letter and your response are not intended to constitute a contract of employment for a definite term. If you accept our offer of employment, you will be an employee at-will, meaning that either you or the Company may terminate our employment relationship at any time for any reason, with or without cause and with or without advance notice. None of the benefits offered to you by the Company create a right to continue in employment for any particular period of time. The terms and conditions of your employment, including without limitation your job title, hours of work, work location, compensation, the stock option plan, and other employee benefits may change over the course of employment at the Company's sole discretion.

Proprietary Rights Agreement.
As a condition of your employment, you are required to sign a Confidential Proprietary Rights and Non-Disclosure Agreement ("Agreement"). The Agreement is enclosed to give you an opportunity to read it carefully prior to your Hire Date. The Agreement must be signed on or before your Hire Date as a condition of employment.

We would like to emphasize the importance we place on the proper treatment of all proprietary information, including that which you may have come into contact with in your prior employment. The Company is extending this offer to you based upon your general skills and abilities, and not your possession of any trade secret, confidential or proprietary information of a former employer. The Company requires that you do not obtain, keep, use for Sarepta's benefit, or disclose this type of information from any prior employers to Sarepta. By accepting this offer, you will also be affirming to the Company that you are not a party to any agreement with a prior employer that would prohibit your employment with us.

Moreover, you agree that during the term of your employment, you will not engage in any other employment, occupation, consulting, or other business activity directly related to the business in which the Company is now involved or becomes involved during the term of your employment, nor will you engage in any other activities that conflict with your obligations to the Company.

Eligibility for Employment.
In compliance with the United States' Citizenship and Immigration Services, Sarepta must verify your identity and eligibility for employment in the United States within 3 business days of your Hire Date. For a list of acceptable documents, please visit http://www.uscis.gov/i-9. Please bring the appropriate documents listed on that form with you when you report for work. Sarepta will not be able to employ you if you fail to comply with this requirement.

In addition, since the Company is a Federal contractor, we participate in e-Verify, an Internet-based system that allows businesses to determine the eligibility of their employees to work in the United States. For more information on this service, please visit http://www.uscis.gov/e-verify.

Change in Control.
Please reference the attached document outlining the terms of the agreement.

Acceptance.
If you wish to accept this offer of employment with Sarepta, please sign below and return one signed copy to me. This offer of employment will expire on Friday, February 10th 2017.

This offer of employment, the Relocation Agreement, and the Confidential Proprietary Rights and Non-Disclosure Agreement (described below) constitute the entire agreement, and supersedes all prior agreements, understanding or statements concerning your employment and all related matters, including, but not limited to, any representations made during your interviews or relocation negotiations, whether written or oral. This offer of employment letter, including, but not limited to, its at-will employment provision, may not be modified or amended, and no breach is regarded as waived, except by a written agreement signed by the Company's CEO and President and you.

We are pleased to welcome you to Sarepta. If you have any questions, please do not hesitate to contact me at 617-274-4076.

Sincerely,

/s/ Edward M. Kaye, MD

Edward M. Kaye, MD
Chief Executive Officer

Enclosures

AGREED TO AND ACCEPTED:
I accept the written terms in this offer of employment letter.

Signature: /s/ Catherine Stehman-Breen Date: February 2, 2017

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.

May 4, 2017

/s/ Edward Kaye, MD
Edward Kaye, MD
President and Chief Executive 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.

May 4, 2017

/s/ Sandesh Mahatme

Sandesh Mahatme
Executive Vice President, Chief Financial Officer and Chief Business 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 March 31, 2017, 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.

May 4, 2017

/s/ Edward Kaye, MD

Edward Kaye, MD
President and Chief Executive 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 March 31, 2017 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.

May 4, 2017

/s/ Sandesh Mahatme
Sandesh Mahatme
Executive Vice President, Chief Financial Officer and Chief Business 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.