Sarepta Therapeutics, Inc.
Form 10-Q
May 05, 2016

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

x QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended March 31, 2016

OR

o 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 x 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 x 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 x	Accelerated filer ¨
Non-accelerated filer ¨ (Do not check if a smaller reporting company)	Smaller Reporting Company ¨

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

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	45,774,907
(Class)	(Outstanding as of April 29, 2016)

SAREPTA THERAPEUTICS, INC.

FORM 10-Q

INDEX

2

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, 2016	As of December 31, 2015
Assets		
Current Assets:		
Cash and cash equivalents	$38,001	$80,304
Short-term investments	91,155	112,187
Accounts receivable	3,990	3,977
Restricted investment	10,695	10,695
Other current assets	16,885	17,380
Total current assets	160,726	224,543
Restricted cash and investments	783	783
Property and equipment, net of accumulated depreciation of $25,826 and $24,594 as of March 31, 2016 and December 31, 2015, respectively	36,982	37,344
Patent costs, net of accumulated amortization of $2,774 and $2,620 as of March 31, 2016 and December 31, 2015, respectively	6,728	6,642
Other non-current assets	8,145	4,470
Total assets	$213,364	$273,782
Liabilities and Stockholders' Equity		
Current Liabilities:		
Accounts payable	$18,673	$20,234
Accrued expenses	24,277	29,053
Current portion of long-term debt	7,604	5,936
Current portion of notes payable	—	2,493
Deferred revenue	3,303	3,303
Other current liabilities	1,303	1,275
Total current liabilities	55,160	62,294
Long-term debt	13,373	14,969
Deferred rent and other	5,869	6,172

Total liabilities	74,402	83,435
Commitments and contingencies (Note 10)		
Stockholders' Equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $.0001 par value, 99,000,000 shares authorized; 45,767,497 and 45,629,529 issued and outstanding at March 31, 2016 and December 31, 2015, respectively	5	5
Additional paid-in capital	1,097,787	1,089,508
Accumulated other comprehensive loss	(5)	(111)
Accumulated deficit	(958,825)	(899,055)
Total stockholders' equity	138,962	190,347
Total liabilities and stockholders' equity	$213,364	$273,782

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited, in thousands, except per share amounts)

	For the Three Months Ended March 31,	
	2016	2015
Revenue from research contracts and other grants	$ —	$ —
Operating expenses:		
Research and development	38,826	39,165
General and administrative	20,876	22,697
Total operating expenses	59,702	61,862
Operating loss	(59,702)	(61,862)
Other income (loss):		
Interest (expense) income and other, net	(68)	303
Total other income (loss)	(68)	303
Net loss	$ (59,770)	$ (61,559)
Other comprehensive income:		
Unrealized gain on short-term securities - available-for-sale	106	78
Total other comprehensive income	106	78
Comprehensive loss	$ (59,664)	$ (61,481)
Net loss per share — basic and diluted	$ (1.31)	$ (1.49)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	45,697	41,324

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,	
	2016	2015
Cash flows from operating activities:		
Net loss	$ (59,770)	$ (61,559)
Adjustments to reconcile net income to cash flows from operating activities:		
Depreciation and amortization	1,397	1,280
Amortization of premium on available-for-sale securities and non-cash interest	242	315
Loss on abandonment of patents	15	131
Stock-based compensation	6,835	14,156
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(13)	—
Net (increase) decrease in other assets	(3,180)	7,413
Net decrease in accounts payable, accrued expenses, deferred revenue and other liabilities	(6,214)	(5,084)
Net cash used in operating activities	(60,688)	(43,348)
Cash flows from investing activities:		
Purchase of property and equipment	(1,168)	(532)
Patent costs	(410)	(391)
Maturity of available-for-sale securities	21,000	44,750
Net cash provided by investing activities	19,422	43,827
Cash flows from financing activities:		
Repayments of long-term debt and notes payable	(2,525)	(25)
Proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program	1,488	431
Net cash (used in) provided by financing activities	(1,037)	406
(Decrease) increase in cash and cash equivalents	(42,303)	885
Cash and cash equivalents:		
Beginning of period	80,304	73,551
End of period	$ 38,001	$ 74,436
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 409	$ 18
Supplemental schedule of non-cash investing activities and financing activities:		
Property and equipment included in accrued expenses	$ 19	$ 45
Patent costs included in accrued expenses	$ 192	$ 100
Shares withheld for taxes	$ 44	$ —
Capitalized interest	$ —	$ 99

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. BUSINESS AND BASIS OF PRESENTATION

Business

Sarepta Therapeutics, Inc. (together with its wholly-owned subsidiaries “Sarepta” or the “Company”) is a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other 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, including its lead DMD product candidate, eteplirsen, designed to skip exon 51.

On April 25, 2016, the U.S. Food and Drug Administration (“FDA”) Peripheral and Central Nervous System Advisory Committee (“PCNSC”) met to review the new drug application (“NDA”) for eteplirsen as a treatment for DMD amenable to exon 51 skipping. The PCNSC voted 6 to 7 against the finding of substantial evidence from adequate and well-controlled studies that show that eteplirsen induces production of dystrophin to a level that is reasonably likely to predict clinical benefit (FDA Question #2). The PCNSC voted 3 to 7, with three abstentions, against finding substantial evidence based on the clinical results of the single historically-controlled study that eteplirsen is effective for treatment of DMD (FDA Question #7). In three additional voting questions, the panel voted 5 to 7, with one abstention, against whether decisions to administer the 6-minute walk test (vs. conclusions that the patient could no longer walk) were sufficiently objective and free of bias and subjective decision-making by patients, their caregivers, and/or health care professionals to allow for a valid comparison between patients in Study201/202 and an external control group (FDA Question #4). The panel voted on the impact of the North Star Ambulatory Assessment with one panel member voting that it strengthened the persuasiveness of the findings in Study 201/202, with five voting that it weakened the persuasiveness, and seven voting that it had no effect (FDA Question #5). The panel also voted on the impact of the other tests of physical performance (e.g., rise time, 10-meter run/walk) on the persuasiveness of the findings in Study 201/202, with the result of one panel member voting that they strengthened the persuasiveness, two voting that they weakened the persuasiveness, and ten voting that they had no effect (FDA Question # 6). The FDA is not bound by the PCNSC’s recommendation but takes its advice into consideration when reviewing New Drug and Biologic License Applications in general. The Prescription Drug User Fee Act action date for eteplirsen remains at May 26, 2016. The Company is also researching and developing therapeutics using its technology for the treatment of drug resistant bacteria and infectious, rare and other human diseases.

The Company has not generated any revenue from product sales to date and there can be no assurance that revenue from product sales will be achieved. Even if it does achieve revenue from product sales, the Company is likely to continue to incur operating losses in the near term.

As of March 31, 2016, the Company had approximately $140.6 million of cash, cash equivalents and investments, consisting of $38.0 million of cash and cash equivalents, $91.2 million of short-term investments and $11.5 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of March 31, 2016 is sufficient to fund its current operational plan for the next twelve months, though it may pursue

additional cash resources through public or private financings, seek additional government funding and establish collaborations with or license its technology to other companies.

Basis of Presentation

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

Estimates and Uncertainties

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

2. RECENT ACCOUNTING PRONOUNCEMENTS

In March 2016, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update (“ASU”) No. 2016-09, “Improvements to Employee Share-Based Payment Accounting”. The amendments in this update simplify several aspects of the accounting for share-based payment transactions, including income tax consequences, classification of awards as either equity or liabilities and classification on the statement of cash flows. ASU No. 2016-09 will be effective for fiscal years beginning after December 15, 2016, with early adoption permitted. As of March 31, 2016, the Company has not elected to adopt this guidance or determined the effect that the adoption of this guidance will have 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. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. As of March 31, 2016, the Company has not elected to adopt this guidance or determined the effect that the adoption of this guidance will have on its consolidated financial statements.

In August 2014, the FASB issued ASU No. 2014-15, “Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern”. This update requires an entity’s management to evaluate whether there are conditions or events, considered in the aggregate, that raise substantial doubt about the entity’s ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued and to provide related disclosures. ASU No. 2014-15 is effective for the annual period ending after December 15, 2016, with early adoption permitted. As of March 31, 2016, the Company has not elected to adopt this guidance, and based on the Company's financial condition as of the date these financial statements were issued or available for issuance, the Company does not expect the adoption of this guidance to have any impact on the current period financial statements.

In May 2014, the FASB issued ASU No. 2014-09, “Revenue from Contracts with Customers (Topic 606)”. This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, “Revenue Recognition”. Under the new guidance, a company is required to recognize revenue when it transfers goods or renders services to customers at an amount that it expects to be entitled to in exchange for these goods or services. The new standard allows for either a full retrospective with or without practical expedients or a retrospective with a cumulative catch upon adoption transition method. This guidance is effective for the fiscal years beginning after December 15, 2016, with early adoption not permitted. In August 2015, the FASB issued ASU No. 2015-14, “Deferral of the Effective Date”, which states that the mandatory effective date of this new revenue standard will be delayed by one year, with early adoption only permitted in fiscal year 2017. As of March 31, 2016, the Company has not yet determined which adoption method it will utilize or the effect that the adoption of this guidance will have on its consolidated financial statements.

3. 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 the valuation techniques it utilizes to determine such fair value:

	Fair Value Measurement as of March 31, 2016			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$178	$178	$—	$ —
Commercial paper	29,971	—	29,971	—
Government and government agency bonds	43,828	—	43,828	—
Corporate bonds	17,356	—	17,356	—
Certificates of deposit	11,343	11,343	—	—
Total assets	$102,676	$11,521	$91,155	$ —

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

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds and certificates of deposit. Money market funds are publicly traded mutual funds and are presented as cash equivalents on the unaudited condensed consolidated balance sheets as of March 31, 2016.

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

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

4. 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, 2016 and December 31, 2015 was approximately 2 and 4 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, 2016		
	Gross	Gross	Fair
Amortized	Unrealized	Unrealized	Market
Cost	Gains	Losses	Value
(in thousands)			

Cash and money market funds	$38,001	$ —	$ —	$38,001
Commercial paper	29,972	—	(1)	29,971
Government and government agency bonds	43,826	3	(1)	43,828
Corporate bonds	17,362	—	(6)	17,356
Total assets	$129,161	$ 3	$ (8)	$129,156
As reported:				
Cash and cash equivalents	$38,001	$ —	$ —	$38,001
Short-term investments	91,160	3	(8)	91,155
Total assets	$129,161	$ 3	$ (8)	$129,156

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

5. 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, 2016	As of December 31, 2015
	(in thousands)	
Manufacturing-related deposits	$12,934	$ 13,070
Prepaid expenses	2,880	3,109
Other	1,071	1,201
Total other current assets	$16,885	$ 17,380

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

	As of March 31,	As of December 31,

	2016	2015
	(in thousands)	
Prepaid clinical expenses	$4,228	$ 4,228
Manufacturing-related deposits	3,676	—
Other	241	242
Total other non-current assets	$8,145	$ 4,470

6. ACCRUED EXPENSES

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

	As of March 31, 2016	As of December 31, 2015
	(in thousands)	
Accrued clinical and preclinical costs	$10,170	$ 9,587
Accrued contract manufacturing costs	5,161	4,830
Accrued employee compensation costs	4,553	8,189
Accrued professional fees	2,957	4,258
Accrued research costs	663	629
Accrued facility-related costs	51	127
Other	722	1,433
Total accrued expenses	$24,277	$ 29,053

7. RESTRUCTURING

On March 8, 2016, the Company announced a long-term plan to consolidate all of the Company's operations to Massachusetts and reduce workforce by approximately 19% as part of a strategic plan to increase operational efficiency. Over the course of the year, the Company plans to close its facility in Corvallis, Oregon, which primarily focused on early-stage research and research manufacturing. As part of the consolidation, research activities and some employees will transition to the Company's facilities in Andover and Cambridge, Massachusetts. The consolidation efforts are planned to occur in four waves - May, October, November and December of 2016, with an estimated completion date of December 30, 2016. The Company estimates restructuring expenses of $1.7 million related to workforce reduction, which will be accrued as earned over the service period for each employee. The Company has not determined the financial impact from facility consolidation but is currently obligated to make $4.3 million of cash lease payments after the estimated completion date of the consolidation plan.

Costs associated with the workforce reduction primarily relate to employee severance and benefits. Facility consolidation costs are primarily associated with non-cancelable lease obligations. For the three months ended March 31, 2016, the Company incurred $0.5 million related to workforce reduction.

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

	For the Three Months Ended

	March 31, 2016		
	(in thousands)		
	Cash	Non-cash (1)	Total
Research and Development	$357	$ 145	$502
General and Administration	31	—	31
Total restructuring costs	$388	$ 145	$533

(1) The non-cash restructuring expense relates to acceleration of stock options.

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

	As of March 31, 2016
	(in thousands)
Restructuring costs incurred during the period	$ 388
Amounts paid during the period	—
Restructuring reserve ending balance	$ 388

8. 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,			
	2016		2015	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options*	1,205,776	$ 11.92	1,607,544	$ 10.35
Restricted stock awards**	25,775	$ 13.71	6,000	$ 13.90

* Included in 2016 stock option grants are 287,500 options with performance conditions that are not currently probable of being achieved. If certain performance milestones are achieved within the required time frame, the Company may recognize up to $3.4 million of stock-based compensation related to these grants when performance is deemed probable. The remaining stock options granted during the periods presented in the table have only service-based criteria and vest over four years.

** Included in 2016 restricted stock awards ("RSA") are 18,755 shares granted to certain employees in lieu of a portion of their 2015 annual bonus payments. These RSA grants have six-month vesting schedules. The remaining RSAs will be fully vested by June 2017.

Stock-based Compensation Expense

For the three months ended March 31, 2016 and 2015, total stock-based compensation expense was $6.7 million and $14.2 million, respectively. Included in the amount for the three months ended March 31, 2015 is $8.7 million of stock-based compensation expense incurred in connection with the resignation of the Company's former Chief Executive Officer ("CEO"). 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	
	2016	2015
	(in thousands)	
Research and development	$2,449	$2,446
General and administrative	4,241	11,710
Total stock-based compensation expense	$6,690	$14,156

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	
	2016	2015
	(in thousands)	
Stock options	$5,698	$13,551
Restricted stock awards	184	42
Stock appreciation rights	115	147
Employee stock purchase plan	693	416
Total stock-based compensation expense	$6,690	$14,156

9. NET LOSS PER SHARE

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

	For the Three Months Ended	
	March 31,	
	2016	2015
	(in thousands, except per share amounts)	
Net loss	$(59,770)	$(61,559)
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	45,697	41,324
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	—
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	45,697	41,324
Net loss per share — basic and diluted	$(1.31)	$(1.49)

* For the three months ended March 31, 2016 and 2015, stock options, RSAs and stock appreciation rights to purchase approximately 7.9 million and 6.9 million shares of common stock, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

10. 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) (“Corban”) by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. The plaintiffs’ consolidated amended complaint, filed on July 21, 2014, sought to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company

between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleged that Sarepta and certain of its officers violated the federal securities laws in connection with disclosures related to eteplirsen, the Company's lead therapeutic candidate for DMD, and sought damages in an unspecified amount. On March 31, 2015, the Court granted Sarepta's motion to dismiss the plaintiffs' amended complaint. On August 12, 2015, the Court denied the plaintiffs' April 30, 2015 motion for leave seeking to file a further amended complaint, and on September 22, 2015, the Court dismissed the case. The plaintiffs filed a Notice of Appeal in the Court of Appeals for the First Circuit on September 29, 2015. On January 27, 2016, the plaintiffs filed a motion to vacate the District Court's order denying leave to amend and dismissing the case, during the pendency of which the plaintiffs' appeal was stayed. On April 21, 2016, the Court denied that motion. A briefing schedule for the plaintiffs' appeal will be set by the First Circuit. An estimate of the possible loss or range of loss cannot be made at this time.

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

In addition, three derivative suits were filed based upon the Company's disclosures related to eteplirsen. On February 5, 2015, a derivative suit was filed against the Company's Board of Directors in the 215th Judicial District of Harris County, Texas (David Smith, derivatively on behalf of Sarepta Therapeutics, Inc., v. Christopher Garabedian et. al, Case No. 2015-06645). The claims allege that Sarepta's directors caused Sarepta to disseminate materially false and/or misleading statements in connection with disclosures concerning the Company's submission of the NDA for eteplirsen. Plaintiff seeks unspecified compensatory damages,

actions to reform and improve corporate governance and internal procedures, disgorgement of profits, benefits and other compensation obtained by the directors, and attorneys' fees. On March 24, 2015, the parties agreed to abate the case pending the resolution of both suits pending in federal court in the District of Massachusetts, Corban and Kader. Additionally, on February 24, 2015, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (Ira Gaines, and the Ira J. Gaines Revocable Trust U/A, on behalf of nominal defendant Sarepta Therapeutics, Inc., vs. Goolsbee et. al., No. 10713). The claims allege that the defendants participated in making material misrepresentations or omissions during the period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of the NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs seek unspecified compensatory damages, punitive damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. On March 26, 2015, the parties agreed to stay the case pending the resolution of Kader, pending in federal court in the District of Massachusetts. On March 29, 2016, the parties submitted a stipulation of dismissal, and the Court approved the dismissal with prejudice as to plaintiffs Ira Gaines, and the Ira J. Gaines Revocable Trust U/A, with each side bearing its own fees, costs and expenses. A third derivative complaint was filed was filed in the U.S. District Court for the District of Massachusetts on March 16, 2016 (Dawn Cherry, on behalf of nominal defendant Sarepta Therapeutics, Inc., vs. Behrens et. al., 1:16-cv-10531-ADB). The claims allege that the defendants authorized the Company to make materially false and misleading statements about the Company's business prospects in connection with its development of eteplirsen from July 10, 2013 to the present. Plaintiffs seek unspecified damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. An estimate of the possible loss or range of loss cannot be made at this time.

Additionally, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et. al vs. Goolsbee et. al., No. 10157). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's former CEO, Christopher Garabedian, was also excessive and such fees were the basis for Mr. Garabedian's not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, among others, includes disgorgement and rescindment of allegedly excessive or unfair payments and equity grants to Mr. Garabedian and the directors, unspecified damages plus interest, a declaration that the Company's Amended and Restated 2011 Equity Plan at the 2013 annual meeting was ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. We have reached an agreement in principle with the parties in the McDonald suit and do not believe that disposition of the McDonald suit will have a material financial impact on the Company.

11. SUBSEQUENT EVENT

On April 29, 2016, the Patent Trial and Appeal Board (the "PTAB") of the United States Patent and Trademark Office ("USPTO") entered a judgment on the motions to end Interference No. 106,007 between U.S. Patent No. 8,455,636 held by the Company (under license from the University of Western Australia) and U.S. Application No. 11/233,495 held by BioMarin Pharmaceutical, Inc. ("BioMarin") (under license from Academisch Ziekenhuis Leiden) 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 U.S. Application No. 11/233,495, with the exception of claim 77; and (ii) cancellation of all claims in the Company's U.S. Patent No. 8,455,636, in each case based on its decision of various motions. Notably, the PTAB granted the Company's motion, with exception to claim 77, asserting that BioMarin's claims in U.S. Application No. 11/233,495 are unpatentable as not being supported by an adequate written description and are not enabled. The PTAB denied the Company's 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 the Company's U.S. Patent No. 8,455,636 and BioMarin's U.S. Application No. 14/248,279. In addition, the PTAB granted BioMarin's motions asserting that the Company's claims in U.S. Patent No. 8,455,636 are unpatentable as being obvious in view of certain prior art or as claiming unpatentable subject matter. The PTAB denied BioMarin's motion to add two additional claims to its U.S. Application No. 11/233,495. This judgment of the PTAB is subject to appeal.

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

This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2015 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "estimate," "could," "continue," "ongoing," "predict," "pote and other similar expressions, as well as variations or negatives of these words. You should read these statements carefully because they discuss future expectations, 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 expectations regarding the timing of research, development, preclinical and clinical trial results, data and analyses relating to the safety profile and potential clinical benefits of our product candidates, including eteplirsen, our phosphorodiamidate morpholino oligomer ("PMO") chemistries, our other PMO-based chemistries and our other RNA-targeted technologies;
- our expectations regarding the Food and Drug Administration's ("FDA") interpretation of our data and information on our product candidates, PMO and PMO-based chemistries and RNA-targeted technologies and the impact on our business of the FDA's interpretations on our FDA submissions (including our investigational new drug applications ("INDs") and new drug applications ("NDAs")), filing decisions by the FDA, potential advisory committee meeting dates and advisory committee recommendations, and FDA product approval decisions and related timelines;
- our ability to respond to FDA requests during the regulatory process for each of our product candidates, including eteplirsen;
- the FDA's pending decision on our eteplirsen NDA by the Prescription Drug User Fee Act ("PDUFA") action date of May 26, 2016 and the impact on our business if eteplirsen does not receive marketing approval by the PDUFA action date, including the possibility that we will have to delay or terminate some of our pre-clinical and clinical studies and cut certain programs from our pipeline of product candidates;
- our investment in and activities in preparation for a potential commercial launch of eteplirsen, including negotiating and entering into commercial and supply contracts, scaling up manufacturing and hiring commercial positions and the impact of winding down or terminating these commitments if the FDA does not approve our eteplirsen NDA;
- our estimates regarding how long our currently available cash, cash equivalents and investments will be sufficient to finance our operations and business plans and statements about our future capital needs;
- our ability to raise additional funds to support our business plans, potential limitations on our ability to raise additional funds if eteplirsen does not receive approval by the PDUFA action date, the possibility that MidCap Financial might take the position that not getting approval by the PDUFA action date is an event of default under our credit and security agreement, and the impact of our credit and security agreement with MidCap Financial on our financial condition and future operations;
- our expectations regarding our ability to become a leading developer and marketer of PMO-based and RNA-targeted therapeutics and commercial viability of our product candidates, chemistries and technologies;
- the potential safety, efficacy, potency and utility of our product candidates, chemistries and technologies in the treatment of Duchenne muscular dystrophy ("DMD") and in rare, infectious and other diseases;
    - our expectations regarding the timing, completion and receipt of results from our ongoing development programs for our pipeline of product candidates including their potential consistency with prior results;
- our ability to effectively manage the clinical trial process for our product candidates on a timely basis, including our ability to conduct a placebo-controlled confirmatory study for eteplirsen in the U.S. using an exon 53-skipping product candidate and any potential delays or changes to this study or our other studies if the FDA does not provide marketing approval for eteplirsen by the PDUFA date;
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our expectations regarding our ability to engage a number of manufacturers with sufficient capability and capacity to meet our manufacturing needs, including with respect to the manufacture of subunits, drug substance ("APIs") and drug product, within the time frames and quantities needed to provide our product candidates, including eteplirsen, to patients in larger scale clinical trials or in potential commercial quantities, and meet regulatory and Company quality control requirements;

- the impact of regulations as well as regulatory decisions by the FDA and other regulatory agencies on our business, including with respect to our eteplirsen NDA submission as well as the development of our product candidates and our financial and contractual obligations;
- our expectations regarding the potential markets for our product candidates;
- our expectations regarding our manufacturing and scale-up techniques and our ability to synthesize and purify our product candidates to adequately support clinical development and potential commercialization;
- the potential acceptance of our product candidates, if introduced, in the marketplace;
- the possible impact of competing products on our product candidates and our ability to compete against such products;
- the impact of potential difficulties in product development, manufacturing, or the commercialization of our product candidates, including difficulties in establishing the commercial infrastructure necessary for the commercialization of eteplirsen;
- our expectations regarding partnering opportunities and other strategic transactions;
- the extent of protection that our patents provide and our pending patent applications may provide, if patents issue from such applications, to our technologies and programs, and our ability to maintain patent protection for our technologies and programs;
- our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;
- our ability to invalidate some or all of the claims of patents issued to competitors and pending patent applications if issued to competitors, and the potential impact of those claims on the potential commercialization of our product candidates;
- our ability to successfully challenge the patent positions of our competitors and successfully defend our patent positions in the actions that the United States Patent and Trademark Office or any appeals court may take or has taken with respect to our patent claims or those of third parties, including with respect to interferences that have been declared between our patents and patent applications held by BioMarin Pharmaceuticals, Inc., relating to eteplirsen and SRP-4053 and our expectations regarding the impact of these interferences on our business plans, including our current commercialization plans for eteplirsen and SRP-4053;
- our ability to operate our business without infringing the intellectual property rights of others;
- our ability to enter into contracts, including collaborations or licensing agreements, with respect to our technology and product candidates, with third parties, including government entities;
- our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;
- the timing and outcomes of ongoing interference proceedings and related appeals;
- the impact of litigation on us, including actions brought by stockholders;
- our ability to attract and retain key employees needed to execute our business plans and strategies and our expectations regarding our ability to manage the impact of any loss of key employees;
- our ability to comply with applicable environmental laws and regulations;
- our expectations relating to potential funding from government and other sources for the development of some of our product candidates;
- the impact of the potential achievement of performance conditions and milestones relating to our restricted stock awards;
- our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements; and
- our succession plan, including the search for a permanent Chief Executive Officer (“CEO”) and the effect that the changes in management could have on the Company, its business plans and its regulatory and clinical discussions and relationships.

All forward-looking statements are based on information available to us on the date of this Quarterly Report on Form 10-Q and we will not update any of the forward-looking statements after the date of this Quarterly Report on Form 10-Q, 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

15

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. Applicable risks and uncertainties include, among others, that: the FDA may further delay our PDUFA action date or may not approve eteplirsen as a DMD therapeutic; we may be delayed or may not be able to comply with the FDA's requests for additional information in connection with our eteplirsen NDA; the additional information and data we collect for eteplirsen may not be consistent with prior data or results; we may be delayed in and may not be able to successfully conduct or obtain positive results in our current and planned clinical trials for eteplirsen and other product candidates in our pipeline; if the FDA does not provide marking approval for eteplirsen by the PDUFA action date, we will need to change our current business plans including by potentially delaying or terminating our commercialization contracts, certain of our pre-clinical programs and clinical studies and reducing our pipeline and workforce; we may not have sufficient funds to execute on our business plans and strategy; we may not be able to obtain regulatory approvals for our product candidates in a timely manner nor achieve commercial viability; we may not be able to incorporate our PMO and other technology into therapeutic commercial products; we may not be able to successfully navigate the uncertainties related to regulatory processes; we may not be able to demonstrate acceptable levels of safety, efficacy and quality in our product candidates through our preclinical and clinical trials; compliance with environmental laws could have a negative impact on our business if we are not able to effectively manage our real estate, manufacturing and other Company operations that may deal with hazardous materials; we rely on third parties to provide service, including the manufacturing of our product candidates, in connection with our preclinical and clinical development programs and commercialization plan and we may not be able to secure the service or quality of service we need from third parties; the pharmaceutical industry is subject to greater government scrutiny and regulation, and we may not be able to respond to changing laws and regulations affecting our industry, including any reforms to the regulatory approval process administered by the FDA or changing enforcement practices related thereto; we may not be able to obtain and maintain patent protection for our product candidates, preserve our trade secrets or prevent third parties from infringing on our proprietary rights; we may not be able to capitalize on our executive team's relationships and expertise to meet our expected timelines for regulatory submissions, clinical development plans and bringing our product candidates to market; we may not be able to hire and retain key personnel or attract qualified personnel, including a permanent full-time CEO; we may not be able to establish and maintain arrangements with third parties who are able to meet manufacturing needs for large-scale clinical trials or potential commercial needs within sufficient timelines or at acceptable costs; competitive products and pricing may have a negative impact on our business; there are uncertainties associated with our future capital needs; we may not be able to raise additional funds to execute our business plans; we may not be able to attract sufficient capital or to enter into strategic relationships; the outcome of our patent interferences, investigations and litigation and associated damages and expenses is uncertain; and those risks and uncertainties discussed in Item 1A "Risk Factors" of this Quarterly Report on Form 10-Q.

Overview

We are a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other 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 DMD drug candidates, including our lead DMD product candidate, eteplirsen, designed to skip exon 51. We are also developing therapeutics using our technology for the treatment of drug resistant bacteria and infectious, rare and other human diseases.

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. Currently, there are no approved

disease-modifying therapies for DMD in the U.S. Eteplirsen is our lead therapeutic candidate for DMD. If we are successful in our development efforts, eteplirsen will address an unmet medical need. We are in the process of conducting or starting several studies for product candidates designed to skip exons 51, 45 and 53 in the U.S. and in Europe. These are comprised of (i) studies to further evaluate eteplirsen that include an open label extension of our Phase IIb study, a confirmatory trial in ambulatory patients, a study on participants with advanced stage DMD and a study with participants with early stage DMD, (ii) a dose-ranging study for our product candidate designed to skip exon 45, (iii) a two-part randomized, double-blind, placebo-controlled, dose titration safety, tolerability and pharmacokinetics study (Part I) followed by an open label efficacy and safety study (Part II) with a product candidate designed to skip exon 53 and (iv) a placebo-controlled confirmatory study with product candidates designed to skip exons 45 and 53 for which we have satisfactorily addressed FDA inquiries on preclinical data relating to the exon 53-skipping product candidate.

On April 25, 2016, the FDA Peripheral and Central Nervous System Advisory Committee (“PCNSC”) met to review the NDA for eteplirsen as a treatment for DMD amenable to exon 51 skipping. The PCNSC voted 6 to 7 against the finding of substantial evidence from adequate and well-controlled studies that show that eteplirsen induces production of dystrophin to a level that is reasonably likely to predict clinical benefit (FDA Question #2). The PCNSC voted 3 to 7, with three abstentions, against finding

substantial evidence based on the clinical results of the single historically-controlled study that eteplirsen is effective for treatment of DMD (FDA Question #7). In three additional voting questions, the panel voted 5 to 7, with one abstention, against whether decisions to administer the 6-minute walk test (vs. conclusions that the patient could no longer walk) were sufficiently objective and free of bias and subjective decision-making by patients, their caregivers, and/or health care professionals to allow for a valid comparison between patients in Study201/202 and an external control group (FDA Question #4). The panel voted on the impact of the North Star Ambulatory Assessment with one panel member voting that it strengthened the persuasiveness of the findings in Study 201/202, with five voting that it weakened the persuasiveness, and seven voting that it had no effect (FDA Question #5). The panel also voted on the impact of the other tests of physical performance (e.g., rise time, 10-meter run/walk) on the persuasiveness of the findings in Study 201/202, with the result of one panel member voting that they strengthened the persuasiveness, two voting that they weakened the persuasiveness, and ten voting that they had no effect (FDA Question # 6). The FDA is not bound by the PCNSC's recommendation but takes its advice into consideration when reviewing New Drug and Biologic License Applications in general. The PDUFA action date for eteplirsen remains at May 26, 2016.

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

We believe we have developed proprietary state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of our product candidates to support clinical development as well as potential 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 mid-to-large scale manufacturing capabilities to support our product candidates.

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

We have not generated any revenue from product sales to date and there can be no assurance that revenue from product sales will be achieved. Even if we achieve revenue from product sales, we are likely to continue to incur operating losses in the near term.

As of March 31, 2016, we had approximately $140.6 million of cash, cash equivalents and investments, consisting of $38.0 million of cash and cash equivalents, $91.2 million of short-term investments and $11.5 million of restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months. As of December 31, 2014, we had completed all development activities under the agreements with the DoD. We are currently exploring possibilities for funding, collaboration and other avenues to support further development of these Ebola, Marburg and influenza product

candidates. Without funding from the U.S. government, we would likely curtail certain infectious disease research and development efforts, though we may pursue additional cash resources through public or private financings, seek additional government funding and establish collaborations with or license our technology to other companies.

The likelihood of our long-term success must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new pharmaceutical products, competitive factors in the marketplace, the risks associated with government sponsored programs and the complex regulatory environment in which we operate. There can be no assurance that we will ever achieve commercialization, significant revenue or profitable operations.

Key Financial Metrics

Revenue

Revenue from Research Contracts and Other Grants. We recognize revenue from research contracts and other grants during the period in which the related expenses are incurred and present such revenue and related expenses on a gross basis in the unaudited condensed consolidated financial statements. Our government contracts are subject to government audits, which may result in catch-up adjustments.

If a technology, right, product or service is separate and independent of our performance under other elements of an arrangement, we defer recognition of non-refundable up-front fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, such up-front fees are deferred and recognized over the period of continuing involvement. As of March 31, 2016, we had deferred revenue of $3.3 million, which represents up-front fees which we may recognize as revenue upon settlement of certain obligations.

Expenses

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

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

Future research and development expenses may increase as our internal projects, such as those for our DMD product candidates, enter or proceed through later stage clinical development. We are currently conducting various clinical trials for eteplirsen, including a confirmatory trial in the U.S. We completed Part I and are conducting Part II of a Phase I/IIa clinical trial for an exon 53-skipping product candidate in the E.U. We have also initiated a dose-ranging study for our exon 45-skipping product candidate in the U.S. We are also planning to initiate a placebo-controlled confirmatory study with product candidates designed to skip exons 45 and 53 in the U.S. and E.U. The remainder of our research and development programs are in various stages of research and preclinical 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 the other risks inherent in the drug development process, we cannot determine the duration or completion costs of current or future clinical stages of any of our product candidates. Similarly, we cannot determine when, if, or to what extent we may generate 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.

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

administrative expenses include an allocation of our facility costs and professional fees for legal, consulting and accounting services.

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

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations is based upon our unaudited condensed consolidated financial statements included elsewhere in this report. The preparation of our unaudited condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States ("U.S. GAAP") requires us to make

estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and related disclosure of contingent assets and liabilities for the periods presented. Some of these judgments can be subjective and complex and, consequently, actual results may differ from these estimates. For any given individual estimate or assumption we make, there may also be other estimates or assumptions that are reasonable. We believe that the estimates and judgments upon which we rely are reasonable based upon historical experience and information available to us at the time when we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our unaudited condensed consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates.

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

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

There have been no material changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2015 filed with the SEC on February 26, 2015.

Results of Operations for the Three and Nine Months Ended March 31, 2016 and 2015

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

	For the Three Months Ended March 31,			
	2016	2015	Change	Change
	(in thousands, except per share amounts)		$	%
Revenue from research contracts and other grants	$—	$—	$—	NA
Operating expenses:				
Research and development	38,826	39,165	(339)	(1)%
General and administrative	20,876	22,697	(1,821)	(8)%
Total operating expenses	59,702	61,862	(2,160)	(3)%
Operating loss	(59,702)	(61,862)	2,160	(3)%
Other income (loss):				
Interest (expense) income and other, net	(68)	303	(371)	(122)%
Net loss	$(59,770)	$(61,559)	$1,789	(3)%
Net loss per share — basic and diluted	$(1.31)	$(1.49)	$0.18	(12)%

Revenue

As of December 31, 2014, we had completed all development activities related to our contracts with the U.S. government. Therefore, no revenue was recognized for the three months ended March 31, 2016 or 2015. The majority

of the revenue under our U.S. government contracts has been recognized as of March 31, 2016 and only revenue for contract finalization, if any, is expected in the future.

Research and Development Expenses

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

	For the Three Months Ended March 31,			
	2016	2015	Change $	Change %
	(in thousands)			
Eteplirsen (exon 51)	$20,012	$21,642	$(1,630)	(8)%
Exon 45	1,432	2,884	(1,452)	(50)%
Exon 53	1,893	927	966	104 %
Other projects	527	592	(65)	(11)%
Internal research and development expenses	14,962	13,120	1,842	14 %
Total research and development expenses	$38,826	$39,165	$(339)	(1)%

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

	For the Three Months Ended March 31,			
	2016	2015	Change $	Change %
	(in thousands)			
Clinical and manufacturing expenses	$22,364	$23,854	$(1,490)	(6)%
Compensation and other personnel expenses	6,432	5,995	437	7 %
Stock-based compensation	2,449	2,446	3	0 %
Professional services	2,400	1,108	1,292	117 %
Facility-related expenses	2,084	2,379	(295)	(12)%
Preclinical expenses	1,084	1,346	(262)	(19)%
Research and other	2,013	2,037	(24)	(1)%
Total research and development expenses	$38,826	$39,165	$(339)	(1)%

Research and development expenses for the three months ended March 31, 2016 decreased by $0.3 million, or 1%, compared with the three months ended March 31, 2015. The decrease was primarily due to a decrease of $1.5 million in clinical and manufacturing expenses, driven by a decrease in manufacturing activities due to timing of raw material purchases offset by increased enrollment in our ongoing clinical trials. The decrease was partially offset by an increase of $1.3 million in professional services primarily due to preparation for the potential regulatory approval of eteplirsen.

General and Administrative Expenses

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

	For the Three Months Ended March 31,				
	2016	2015	Change $	Change %	
	(in thousands)				
Compensation and other personnel expenses	$7,869	$3,698	$4,171	113	%
Professional services	5,796	3,869	1,927	50	%
Stock-based compensation	4,241	3,047	1,194	39	%
Estimated severance expenses	—	9,287	(9,287)	(100	)%
Other	2,970	2,796	174	6	%
Total general and administrative expenses	$20,876	$22,697	$(1,821)	(8	)%

General and administrative expenses for the three months ended March 31, 2016 decreased by $1.8 million, or 8%, compared with the three months ended March 31, 2015. This was primarily due to a decrease of $9.3 million in severance expenses related to the resignation of our former CEO on March 31, 2015 partially offset by increases of $4.2 million in compensation and other personnel expenses and $1.2 million in stock-based compensation primarily driven by an increase in headcount as well as $1.9 million in professional services primarily driven by preparation for the potential product launch for eteplirsen.

Interest (Expense) Income and Other, Net

For the three months ended March 31, 2016, interest expense and other, net was less than $0.1 million. For the three months ended March 31, 2015, interest income and other, net was $0.3 million. The unfavorable change was primarily driven by interest expense incurred in connection with the $20.0 million senior secured term loan.

Liquidity and Capital Resources

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

	As of March 31, 2016	As of December 31, 2015	Change $	Change %
	(in thousands)			
Financial assets:				
Cash and cash equivalents	$38,001	$80,304	$(42,303)	(53)%
Short-term investments	91,155	112,187	(21,032)	(19)%
Restricted cash and investments	11,478	11,478	—	(—)%
Total cash, cash equivalents and investments	$140,634	$203,969	$(63,335)	(31)%
Borrowings:				
Long-term debt	$20,977	$20,905	$72	0%
Notes payable	—	2,493	(2,493)	(100)%
Total borrowings	$20,977	$23,398	$(2,421)	(10)%
Working capital				
Current assets	$160,726	$224,543	$(63,817)	(28)%
Current liabilities	55,160	62,294	(7,134)	(11)%
Total working capital	$105,566	$162,249	$(56,683)	(35)%

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

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

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

Our cash requirements are expected to continue to increase as we advance our research, development and commercialization programs and 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 technology. 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,			
	2016	2015	Change $	Change %
	(in thousands)			
Cash provided by (used in)				
Operating activities	$(60,688)	$(43,348)	$(17,340)	40 %
Investing activities	19,422	43,827	(24,405)	(56)%
Financing activities	(1,037)	406	(1,443)	(355)%
(Decrease) increase in cash and cash equivalents	$(42,303)	$885	$(43,188)	(4,880)%

Operating Activities. The increase in cash used in operating activities of $17.3 million for the three months ended March 31, 2016 compared with the three months ended March 31, 2015 was primarily due to unfavorable changes in operating assets and liabilities of $11.7 million and a decrease in non-cash adjustments of $7.4 million partially offset by a decrease of $1.8 million in net loss primarily driven by decreases in research and development and general and administrative expenses.

Investing Activities. The decrease in cash provided by investing activities was $24.4 million for the three months ended March 31, 2016 compared with the three months ended March 31, 2015 was primarily driven by a decrease in maturity of available-for-sale securities of $23.8 million.

Financing Activities. The cash used in financing activities was $1.0 million for the three months ended March 31, 2016. The cash provided by financing activities was $0.4 million for the three months ended March 31, 2015. The unfavorable change of $1.4 million was primarily driven by a payment of $2.5 million for the promissory note related to the acquisition of our Andover, Massachusetts facility in May 2014 partially offset by an increase of $1.1 million in proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program.

Milestone Obligations

We are obligated to make up to $99.9 million of future development, upfront royalty and commercial milestone payments associated with some of our collaboration and license agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or commercial milestones. Because the achievement of these milestones had not occurred as of March 31, 2016, such contingencies have not been recorded in our financial statements. Amounts related to contingent milestone payments are not considered contractual obligations as they are contingent on the successful achievement of certain development, regulatory approval and commercial milestones.

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, 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, 2016.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, government and government agency bonds and high-grade corporate bonds with maturities of three years or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of March 31, 2016, we had approximately $140.6 million of cash, cash equivalents and investments, comprised of $38.0 million of cash and cash equivalents, $91.2 million of short-term investments and $11.5 million of restricted cash and investments. Our cash equivalents and short-term investments consist of commercial paper, government and government agency debt securities, corporate bonds and money market investments. 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, 2016, 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, 2016, under the supervision and with the participation of our management, including our interim Chief Executive Officer ("CEO") and our Chief Financial Officer ("CFO"), 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 interim CEO and our CFO, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of March 31, 2016, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended March 31, 2016, there were no changes in the Company's internal controls over financial reporting that have materially affected or are reasonably likely to materially affect the Company's internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

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) ("Corban") by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. The plaintiffs' consolidated amended complaint, filed on July 21, 2014, sought to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleged that Sarepta and certain of its officers violated the federal securities laws in connection with disclosures related to eteplirsen, the Company's lead therapeutic candidate for Duchenne muscular dystrophy, and sought damages in an unspecified amount. On March 31, 2015, the Court granted Sarepta's motion to dismiss the plaintiffs' amended complaint. On August 12, 2015, the Court denied the plaintiffs' April 30, 2015 motion for leave seeking to file a further amended complaint, and on September 22, 2015, the Court dismissed the case. The plaintiffs filed a Notice of Appeal in the Court of Appeals for the First Circuit on September 29, 2015. On January 27, 2016, the plaintiffs filed a motion to vacate the District

Court's order denying leave to amend and dismissing the case, during the pendency of which the plaintiffs' appeal was stayed. On April 21, 2016, the Court denied that motion. A briefing schedule for the plaintiffs' appeal will be set by the First Circuit.

Another purported class action complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 (Kader v. Sarepta et.al 1:14-cv-14318) ("Kader"), asserting that the Company and certain of its officers violated Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5. The plaintiffs' amended complaint, filed on March 20, 2015, alleged that the defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of a new drug application ("NDA") for eteplirsen and the likelihood of the Food and Drug Administration ("FDA") accepting the NDA based on that data. The plaintiffs sought compensatory damages and fees. On April 5, 2016, the Court granted Sarepta's motion to dismiss the amended complaint. On April 8, 2016, the plaintiffs filed a motion for leave to further amend the complaint, which Sarepta opposed on April 22, 2016. That motion remains pending.

In addition, three derivative suits were filed based upon the Company's disclosures related to eteplirsen. On February 5, 2015, a derivative suit was filed against the Company's Board of Directors in the 215th Judicial District of Harris County, Texas (David Smith, derivatively on behalf of Sarepta Therapeutics, Inc., v. Christopher Garabedian et. al, Case No. 2015-06645). The claims allege that Sarepta's directors caused Sarepta to disseminate materially false and/or misleading statements in connection with disclosures concerning the Company's submission of the NDA for eteplirsen. Plaintiff seeks unspecified compensatory damages, actions to reform and improve corporate governance and internal procedures, disgorgement of profits, benefits and other compensation obtained by the directors, and attorneys' fees. On March 24, 2015, the parties agreed to abate the case pending the resolution of both suits pending in federal court in the District of Massachusetts, Corban and Kader. Additionally, on February 24, 2015, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (Ira Gaines, and the Ira J.

Gaines Revocable Trust U/A, on behalf of nominal defendant Sarepta Therapeutics, Inc., vs. Goolsbee et. al., No. 10713). The claims allege that the defendants participated in making material misrepresentations or omissions during the period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of the NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs seek unspecified compensatory damages, punitive damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. On March 26, 2015, the parties agreed to stay the case pending the resolution of Kader, pending in federal court in the District of Massachusetts. On March 29, 2016, the parties submitted a stipulation of dismissal, and the Court approved the dismissal with prejudice as to Plaintiffs Ira Gaines, and the Ira J. Gaines Revocable Trust U/A, with each side bearing its own fees, costs and expenses. A third derivative complaint was filed was filed in the U.S. District Court for the District of Massachusetts on March 16, 2016 (Dawn Cherry, on behalf of nominal defendant Sarepta Therapeutics, Inc., vs. Behrens et. al., 1:16-cv-10531-ADB). The claims allege that the defendants authorized the Company to make materially false and misleading statements about the Company's business prospects in connection with its development of eteplirsen from July 10, 2013 to the present. Plaintiffs seek unspecified damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees.

Additionally, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et. al vs. Goolsbee et. al., No. 10157). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's former 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. We have reached an agreement in principle with the parties in the McDonald suit and do not believe that disposition of the McDonald suit will have a material financial impact on the Company.

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 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

If the U.S. Food and Drug Administration ("FDA") makes a decision on eteplirsen that is consistent with the votes taken by the Peripheral and Central Nervous System Advisory Committee ("PCNSC") on April 25, 2016 and does not approve our eteplirsen new drug application ("NDA") by the currently planned Prescription Drug User Fee Act ("PDUFA") action date, or at all, our business may be negatively impacted and we may suffer financial losses in connection with delaying or terminating contracts, manufacturing commitments and employees hired in connection with our activities

in preparation for a potential commercial launch as well as potentially delaying or terminating programs in our pipeline including pre-clinical and clinical studies.

On April 25, 2016, the PCNSC, among other votes, voted 6 - 7 against the finding of substantial evidence from adequate and well controlled studies that show that eteplirsen induces production of dystrophin to a level that is reasonably likely to predict clinical benefit (FDA Question #2). The advisory committee also voted 3 – 7, with three abstentions, against finding substantial evidence based on the clinical results of the single historically controlled study (Study 201/202) that eteplirsen is effective for treatment of DMD (FDA Question #7). While the FDA is not bound by the PCNSC's determination, the FDA takes its advice into consideration when reviewing New Drug and Biologic License Applications. Given the potential commercialization timelines, we commenced, and for the most part completed, certain pre-launch and commercialization investments and activities including, but not limited to, negotiating and entering into supply and other commercial agreements, scaling up manufacturing and hiring certain positions needed for pre-launch and commercial activities and operations. If the FDA delays or does not provide approval for our eteplirsen NDA by the currently planned PDUFA date of May 26, 2016, or at all, or we need to delay or discontinue our development and commercialization plans for eteplirsen for other reasons, our business and the development of our follow-on Duchenne muscular dystrophy ("DMD") product candidates may be negatively impacted and we may incur financial losses in connection with delaying, winding down or terminating the investments, contracts and commitments we entered into for the purpose of positioning ourselves for

a commercial launch of eteplirsen. Additionally, if the approval for eteplirsen is significantly delayed or not obtained, we will need to re-evaluate our pipeline and determine which of our programs will be delayed or terminated including with respect to our pre-clinical and clinical studies.

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

Our most advanced product candidate is eteplirsen for which the FDA is reviewing an NDA for which it held an advisory committee meeting on April 25, 2016 and which has a PDUFA action date of May 26, 2016. Eteplirsen is being evaluated in several clinical studies, including a confirmatory Phase III clinical trial. The exon 53-skipping product candidate, which we are working on with the SKIP-NMD consortium, is currently in the clinic in EU. The Part I dose-titration portion of this Phase I/IIa study has been completed and Part II of the study is ongoing. We are also in the process of conducting a placebo-controlled dose titration study for our exon 45-skipping product candidate. Additionally, we are working towards initiating a clinical trial in the U.S. and the E.U. for exon 45- and 53-skipping product candidates and have satisfactorily responded to the FDA's inquiries on pre-clinical data for this study relating to our exon-53 product candidate. 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, eteplirsen, 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, preclinical development or inactive. Assuming eteplirsen is approved, 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 eteplirsen and other exon-skipping candidates as part of our larger pan-exon strategy in DMD, our infectious disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates or successfully obtain approvals needed to market them. In particular, if the FDA does not approve eteplirsen, we will need to re-evaluate our pipeline and which programs we are able to proceed with as well as which pre-clinical and clinical studies we may need to change, delay or terminate based on the FDA's decision.

Our RNA-targeted antisense technology has not been incorporated into a therapeutic commercial product and is still at an early stage of development.

Our RNA-targeted platforms, utilizing proprietary phosphorodiamidate morpholino oligomer ("PMO")-based technology, have not been incorporated into a therapeutic commercial product and are still at an early stage of development. This technology is used in all of our product candidates, including eteplirsen. Although we have conducted and are in the process of conducting clinical studies with eteplirsen, an exon 45-skipping product candidate and an exon 53-skipping product candidate and preclinical studies with our other product candidates that use our PMO-based antisense technology, additional studies may be needed to determine the safety and efficacy of our PMO-based antisense technology. In addition, nonclinical models used to evaluate the activity and toxicity of product candidate compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease. As such, there may be substantially different results observed in clinical trials from those observed in preclinical studies. Any failures or setbacks in developing or utilizing our PMO-based technology, including adverse effects in humans, could have a detrimental impact on our product candidate pipeline and our ability to maintain and/or enter into new corporate collaborations regarding these technologies, which would negatively affect our business and financial condition.

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

To date, we have been granted orphan drug designation under the Orphan Drug Act by the FDA for two of our product candidates in DMD (including eteplirsen), AVI-7537 for the treatment of Ebola virus and AVI-7288 for the treatment of the Marburg virus. Upon approval from the FDA of an NDA, products granted orphan drug status are generally provided with seven years of marketing exclusivity in the U.S., meaning the FDA generally will not approve applications for other product candidates for the same orphan indication that contain the same active ingredient. Even if we are the first to obtain approval of an orphan product and are granted exclusivity in the United States, there are limited circumstances under which a later competitor product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to our product or due to an inability to assure a sufficient quantity of the orphan drug.

We also have been granted orphan medicinal product designations in the E.U. for two of our product candidates in DMD (including eteplirsen). Product candidates granted orphan status in Europe can be provided with up to 10 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 may 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.

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

Even if we receive regulatory approvals for any of our product candidates, it is possible that their commercialization may be delayed or they may not become commercially viable products.

Even if a product candidate receives regulatory approval, the product may not gain market acceptance among physicians, patients, healthcare or third-party payers or the medical community, which could limit commercialization of the product. Assuming that any of our product candidates receives the required regulatory approvals, timing and success of commercialization will depend on a number of factors, including but not limited to the following:

- the terms of the FDA's package insert requirements and the time it would take the Company to produce the package insert and comply with any related FDA requirements;
- demonstration and/or confirmation of clinical efficacy and safety and acceptance of the same by the medical community;
- cost-effectiveness of the product;
- the availability of adequate reimbursement by third parties, including government payers such as the Medicare and Medicaid programs, managed care organizations and private health insurers;
- the product's potential advantage over alternative or competitive treatment methods;
- whether the product can be manufactured in commercial quantities and at acceptable costs;
- marketing and distribution support for the product;
- any exclusivities or patent rights applicable to the product;
- the market-size for the product which may be different than expected and may be limited or otherwise impacted by the FDA approved package insert for a product; and
- our ability to achieve and sustain profitability, which may not occur if we are unable to develop and commercialize any of our product candidates, development is delayed or sales revenue from any product candidate that receives

marketing approval is insufficient.

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

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by applicable local, regional and national regulatory authorities and regulations may differ from jurisdiction to jurisdiction. In the United States, 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 United States 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, including eteplirsen, on an accelerated approval (e.g., under the Food and Drug Administration Safety and Innovation Act of 2012 ("FDASIA")) or any other basis, in any jurisdiction, including in the United States, cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our preclinical, clinical, Chemistry, Manufacturing and Controls ("CMC") and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for submissions, advisory committee panels, filings or approvals. The FDA could disagree with our beliefs, interpretations and conclusions regarding data we submit in connection with an NDA submission, including the eteplirsen NDA, or other product candidates, and may delay, reject or refuse to file or approve any NDA submission we make or provide a complete response letter until we meet their additional requirements, if ever. In addition, an advisory committee could determine our data are insufficient to provide a positive recommendation for approval of any NDA we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA submission, the FDA could still deny approval of our product candidates based on their review of the data or other factors. Each of these risks all apply to our eteplirsen NDA which is the only NDA we have submitted to the FDA for review to date.
- The regulatory approval process for product candidates targeting orphan diseases, such as DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, and novel endpoints, such as natural history data and dystrophin measures, is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. We cannot be sure that any of our product candidates, including eteplirsen, will qualify for accelerated approval under FDASIA or any other expedited development, review and approval programs, or that, if a drug does qualify, that the product candidates will be approved, will be accepted as part of any such program or that the review time will be shorter than a standard review. As a result of uncertainty in the approval process, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned INDs and NDAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (e.g., additional patient muscle biopsies and dystrophin analyses), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, an advisory committee or regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could negatively impact the review of our NDA or result in a decision by the Company not to proceed with the development of a product candidate or an NDA submission for a product candidate based on feedback from regulators. For example, in reviewing the dystrophin data and analysis that we submitted for eteplirsen, the FDA previously expressed concerns with dystrophin as a surrogate endpoint and requested an independent assessment of dystrophin positive fibers measured in our eteplirsen Phase IIb study, which we provided. The FDA has also requested natural history data to better evaluate the ongoing clinical results of our eteplirsen 201/202 study, which we have also provided. Any material inconsistencies between our existing data and analysis and any new analysis and additional data we provide to the FDA, including the independent assessment of dystrophin positive fibers, safety data, natural history and data from a fourth biopsy that we have provided, could negatively impact the review of our eteplirsen NDA submission.

While our studies demonstrate statistical significance, the FDA may not consider our six-minute walk test ("6MWT") results, including our comparison of our 6MWT results to matched external natural history data, or, to the extent the FDA considers dystrophin a relevant biomarker, the dystrophin production observed in our studies, as demonstration of, or reasonably likely to predict a clinical benefit. Additionally, the FDA may determine, after evaluating the totality of our data and analysis package for a product candidate, or receiving the vote of an advisory committee, that such package does not support an NDA approval.

- 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 requests in a timely and satisfactory manner could significantly delay or negatively impact our placebo-controlled confirmatory study timelines and/or the development plans we have for the exon 53- and exon 45-skipping product candidates. Responding to requests from regulators and meeting requirements for clinical studies, submissions, filings, advisory committees and approvals may require substantial personnel, financial or other resources, which, as a small pre-commercial biopharmaceutical company, we may not be able to obtain in a timely manner or at all. In addition, our ability to respond to requests from regulatory authorities that involve our agents, third-party vendors and associates may be complicated by our own limitations and those of the parties we work with. For example, changes to CMC processes for the production of eteplirsen may require coordination with our third-party manufacturers, which may or may not be limited in their abilities to execute such regulatory requests. 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 related to clinical trial design and the timing of regulatory decisions with respect to any NDA submissions.

Due to the above factors, among others, our product candidates could take a significantly longer time to gain regulatory approval than we expect, or may never gain regulatory approval, which would delay or eliminate any potential commercialization or product revenue for us and result in a material adverse effect on the Company that could involve changes, delays in or terminations of programs in our pipeline, delays or terminations of pre-clinical and clinical studies, termination of contracts related to the development of our product candidates and potential commercialization of eteplirsen 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., limiting labeling and indications requested by us for a product candidate) may no longer make commercialization of a product candidate desirable for us from a business perspective, which could lead us to decide not to commercialize a product candidate.

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

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

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive preclinical and clinical studies that the product candidate is safe and effective in humans. Ongoing and future preclinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. Furthermore, success in preclinical and early clinical trials does not ensure that the subsequent trials we plan to conduct will be successful, nor does it predict final results of a confirmatory trial. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, then the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld. For example, in 2012, we completed Study 201, a U.S.-based Phase IIb 12-person clinical trial for eteplirsen at 30 mg/kg and 50 mg/kg. Following completion of this study, we initiated Study 202, an ongoing open label extension study with the same participants from Study 201. These trials were initiated, in part, to further demonstrate efficacy and safety, including the production of dystrophin, and explore and identify a more consistently effective dose that may be more appropriate for future clinical trials. While Studies 201 and 202 demonstrated dystrophin

production based on the measurements taken at weeks 24 and 48, respectively, and 6MWT results reported for weeks 62, 74, 84, 96 and 120 supported stabilization of disease progression, we cannot provide assurances that data from the ongoing open label extension study will continue to be positive or consistent through the study periods or that the interpretation by regulators, such as the FDA, of the data we collect for our product candidates, including for eteplirsen, will be consistent with our interpretations. For example, on July 10, 2014, we announced that the 6MWT results for week 144 in Study 202 showed a change in decline from 5%, which was observed prior to 144 weeks, to approximately 8.5%. Additionally, on January 12, 2015, we announced results for week 168 in Study 202, which showed continued ambulation across all patients evaluable on the test, however, patients showed a decline in distance walked on this measure since the week 144 time point. Further, on October 1, 2015 we announced additional clinical efficacy and safety data that demonstrated that (i) eteplirsen provided a statistically significant advantage of 151 meters in the ability of study participants to walk at three years versus an untreated external DMD control, (ii) eteplirsen-treated patients (n=12) experienced a slower rate of decline through week 192 versus untreated external DMD controls and (iii) the eteplirsen safety profile remained consistent with prior results. In January 2016, the FDA made public our eteplirsen Briefing Document Addendum (the “January 2016 Addendum”), which disclosed that at four years, 10 out of 12 patients on

eteplirsen remained ambulatory while 10 out of 13 untreated patients in the external control had lost ambulation (one patient in the external control was still ambulatory at year four, while two patients in the external control were missing data at four years), a statistically significant difference. In addition, the January 2016 Addendum disclosed a statistically significant advantage of 162 meters in the ability of study participants to walk (as measured by the 6MWT) at four years.

If we do not obtain the required approvals to initiate the confirmatory trial for eteplirsen using our exon 45- and 53-skipping product candidates, the data from the confirmatory studies for eteplirsen do not produce the safety and efficacy data required by the FDA for obtaining or maintaining marketing approval, or the FDA does not accept the results of our eteplirsen confirmatory studies as supporting evidence of efficacy, we may need to continue working with the FDA on the design and subsequent execution of any further studies or analysis we plan to conduct or that may be required to obtain and maintain approval of eteplirsen or our other DMD product candidates. Any significant delays or negative developments in the confirmatory studies for eteplirsen could delay or otherwise negatively impact our development plans for our follow-on DMD product candidates, including potentially result in terminations of such programs. For example, in October 2014, we received meeting minutes from a Type B pre-NDA meeting that took place in September 2014 in which the FDA provided updated guidance regarding the information to be provided as part of, or at the time of, our NDA submission for eteplirsen. The guidance stated that the FDA was requiring additional data as part of the NDA submission, including the results from an independent assessment of dystrophin images, the 168 week clinical data from Study 202, and additional safety data from new patients exposed to eteplirsen, specifying the minimum number of patients and minimum duration of exposure. Additionally, the guidance also required patient-level natural history data to be obtained by us from independent academic institutions and requested MRI data from a recent study conducted by an independent group. Although we have provided data and information responsive to the FDA's requests and continue to work to provide the FDA, our additional data and information may not support or result in the approval of our eteplirsen NDA submission.

We currently rely on third parties in the manufacturing process to produce our product candidates and our dependence on these parties, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet actual clinical or commercial product demand may impair the advancement of our 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 our product candidates in the quantities needed to conduct our research and development programs, supply clinical trials or meet commercial demand. 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 our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of our product candidates which creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require. Any interruption of the development or operation of those facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available 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 have our product candidates manufactured in sufficient quality and quantity required for planned preclinical testing, clinical trials and potential commercial use would be adversely affected.

Sarepta, through its third party manufacturers, has produced or is in the process of producing clinical and commercial supply, including for eteplirsen, based on its current understanding of market demands and planned clinical studies. In light of the limited number of third parties with the expertise to produce 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 our product candidates to meet demands that exceed our clinical or commercial assumptions. Further, 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 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 regulatory approval process and potential commercialization. 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 of any of our product candidates.

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

Our contract manufacturers are required to produce our materials, APIs and drug products under current Good Manufacturing Practice regulations ("cGMP"). We and our contract manufacturers are subject to periodic unannounced inspections by the FDA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations and corresponding foreign requirements. We do not have control over a third-party manufacturer's compliance with these regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of our product candidates in a compliant manner on the schedule we require for clinical trials or for potential commercial use. The failure to achieve and maintain high quality compliance, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in patient injury or death or product recalls. Any difficulties or delays in our contractors' manufacturing and supply of product candidates, or any failure of our contractors to maintain compliance with the applicable regulations and requirements could increase our costs, make us postpone or cancel clinical trials, prevent or delay regulatory approvals by the FDA and corresponding state and foreign authorities, prevent the import and/or export of our products, cause us to lose revenue, result in the termination of the development of a product candidate, or have our product candidates recalled or withdrawn from use.

We may not be able to successfully scale up manufacturing of 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 delay or prevent us from developing or commercializing our product candidates.

As we prepare for larger and later stage clinical trials for our product candidates and the potential commercialization of eteplirsen, we are working to increase future manufacturing capacity and scale up production of some of the components of our drug products. During 2016, our focus remains on (i) achieving larger-scale manufacturing capacity for eteplirsen 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 or is cost-effective, or in a time frame required to meet our timelines for clinical trials, potential commercialization and other business plans, or at all. cGMP 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 a product candidate itself or the 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 product and demonstrate stability of product candidates for use in late stage clinical trials (and any subsequent drug products for commercial use), our analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our analytical methods or demonstrate adequate purity, stability or comparability of the product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our analytical methods or to demonstrate adequate purity, stability or comparability, the development of our product candidates and regulatory approval or commercial launch for any resulting drug products 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 improvements in the manufacturing and scale-up processes for 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 our product candidates could cause significant delays in our business plans or prevent

commercialization of our product candidates.

We are winding down our expired U.S. government contract, and 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 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 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 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;
    - participant enrollment and retention 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. In addition, terms may vary significantly among different trial sites and CROs and may subject 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 $59.7 million for the three months ended March 31, 2016. Our accumulated deficit was $958.8 million as of March 31, 2016. Substantially all of our revenue to date has been derived from research and development contracts with the DoD, the last of which expired in July 2014. We have not yet generated any revenue from product sales and have generally incurred expenses related to research and development of our technology and product candidates, from general and administrative expenses that we have incurred while building our business infrastructure. We anticipate that our expenses will increase substantially if and as we:

- continue our research, preclinical 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;
-

establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;

- 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.

Our ability to achieve and maintain profitability depends on various factors including our ability to raise additional capital, partner with third parties for one or more of our programs, complete development of our product candidates, obtain regulatory approvals and market our approved products, if any. It is uncertain when, if ever, we will become profitable and if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of the Company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations.

We will need additional funds to conduct our planned research, development and manufacturing efforts. If we fail to attract significant capital on acceptable terms or fail to enter into strategic relationships, we may be unable to continue to develop our product candidates.

We will likely require additional capital from time to time in the future in order to continue the development of product candidates in our pipeline and to expand our product portfolio. The actual amount of funds that we may need will be determined by many factors, some of which are beyond our control. These factors include the success of our research and development efforts, the status of our preclinical 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. In addition, if the FDA delays or ultimately denies approval of our eteplirsen NDA, raising additional funds may be difficult. 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. For example, on October 9, 2015, we sold 3,250,000 shares of our common stock in an underwritten public offering at a price to the public of $39.00 per share. Additional debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

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 preclinical 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, which may be increased by an additional $20.0 million upon the acceptance by the FDA of the NDA for eteplirsen. This indebtedness could have important consequences, including:

- requiring the Company to maintain pledged cash in favor of MidCap Financial equal to not less than the lesser of the outstanding term loans or (a) $15.0 million prior to the increase in the term loan by an additional $20.0 million and (b) $30.0 million thereafter;

- 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. While we do not believe that the failure of the FDA to approve eteplirsen by the PDUFA action date of May 26, 2016 would constitute a material adverse change as defined in the credit and security agreement, it is possible that MidCap Financial could take a different position that such a result constitutes a material adverse change. If MidCap were to prevail on this position and declare an event of default, MidCap Financial would have the right to increase the existing interest rate by an incremental three percent per annum and to accelerate the maturity of all principal and accrued interest under the credit and security agreement unless the event of default is waived by MidCap Financial. In addition, if we incur additional indebtedness, the risks related to our business and our ability to service our indebtedness would increase.

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 United States. 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, assumptions in the valuation of stock-based compensation and accounting for and valuation of liability classified warrants. 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 of 1986, as amended, 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 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.

We rely on third parties to provide services in connection with our preclinical 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 preclinical 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.

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.

Our former Chief Executive Officer ("CEO") and President resigned on March 31, 2015 and we have appointed an interim CEO. No assurance can be made as to when we will hire a permanent CEO. The existing management team is actively managing the business in accordance with a business strategy approved by the board of directors.

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

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

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

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

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

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

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

We may not be able to obtain and maintain patent protection for our product candidates necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. For example, in July 2014, the Patent Trial and Appeal Board (the “PTAB”) of the United States Patent and Trademark Office (“USPTO”) declared patent interferences between certain patents held by Sarepta (under license from the University of Western Australia, “UWA”) and patent applications held by BioMarin (under license from Academisch

Ziekenhuis Leiden, "AZL") related to exon 51 and exon 53 skipping therapies designed to treat DMD. In particular, the PTAB declared Interference No. 106,008, which identifies Sarepta's/UWA's U.S. Patent Nos. 7,807,816 and 7,960,541, both covering eteplirsen, 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 eteplirsen, 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 eteplirsen 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 our product candidate, eteplirsen, 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 No. 16-1937. 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. 14/198,992 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. This judgment of the PTAB is subject to appeal. We cannot make any assurances about the outcome of the remaining proceeding (Interference No. 106,007) or appeals of any of these three interferences. Any additional adverse rulings, which, in the case of Interference No. 106,008 concerning eteplirsen 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 interferences and related appeals are not in our favor, then the Sarepta/UWA patents involved in these 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 candidates, which may have a material negative impact on our business plans. In addition, if final resolution of the interferences or related appeals are not in our favor, the USPTO may issue the BioMarin/AZL patent applications resulting in the grant of one or more patents that may provide a basis for BioMarin to allege that our product candidates, eteplirsen and/or SRP-4053, infringe such patents. In addition, these interferences, 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 United States 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 eteplirsen infringes on such claims. Because we have not yet initiated an invalidation proceeding in these countries, the outcome and timing of any such proceeding cannot be predicted or determined as of the date of this report.

As a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection or impose compulsory licenses for disease treatments that prove successful. Additionally, jurisdictions other than the United States might have less restrictive patent laws than the United States, 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, or 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 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 our product candidates in important commercial markets.

If 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 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, which is developing competitive pipeline products, has rights to patent claims that, absent a license, may preclude us from commercializing eteplirsen 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 eteplirsen in a European country where BioMarin has a patent corresponding to EP 1619249 would infringe on such patent. Both we and BioMarin have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other’s briefs. BioMarin filed arguments with the EPO in response to Sarepta’s previously filed briefs. The Opposition Division decision, if maintained at the appeals level, could have a substantial negative effect on our business and leaves open the possibility that BioMarin or other parties that have rights to such patent could assert that our product candidate, eteplirsen, 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 eteplirsen and other therapeutic candidates could be materially impaired. Moreover, our ability to commercialize eteplirsen in a European country where BioMarin has a patent related to EP 1619249 while the appeal process remains ongoing before the EPO Board of Appeals could be materially impaired. In addition, we are aware of various divisional applications relating to EP 1619249 that are being pursued by BioMarin, which are pending and in

some cases are granted. Any of these granted patents can also materially impair our ability to commercialize eteplirsen or our other therapeutic candidates, such as SRP-4045 and SRP-4053.

We are also aware of existing patent claims BioMarin is pursuing in the United States, 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 eteplirsen 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 our product candidate, eteplirsen, infringes such granted claims. These patent claims may materially impair our ability to commercialize eteplirsen.

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 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 our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals, Inc.), Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S) and Nippon Shinyaku Co. Ltd. share a focus on RNA-targeted drug discovery and development. Competitors with respect to our exon-skipping DMD program, or eteplirsen, include BioMarin (which acquired Prosensa), Nippon Shinyaku, Daiichi Sankyo and Shire plc; and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs. Additionally, several companies have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Pfizer, Inc., Bristol-Myers Squibb, Biogen Idec, Inc., Ionis Pharmaceuticals, Inc., Alexion Pharmaceuticals, Inc., Sanofi, Eli Lilly, Alnylam, Moderna Therapeutics, Inc., Summit plc, Akashi, Catabasis, and Oxford University. Although BioMarin received a complete response letter for KyndrisaTM (drisapersen) for the treatment of DMD amenable to exon 51 skipping on January 14, 2016, BioMarin continues to be a competitor for us on the development of DMD exon-skipping product candidates. BioMarin announced that its ongoing Kyndrisa extension studies will continue, as will the ongoing clinical trials for other exon-skipping oligonucleotides, BMN 044, BMN 045 and BMN 053, while BioMarin is exploring next steps for this application. If BioMarin is successful in obtaining regulatory approval for any of its exon-skipping 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 our product candidates, impact the regulatory approval process for our product candidates that are more effective than our product candidates or that would render our technology obsolete or noncompetitive. Our competitors, including BioMarin, may, among other things:

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

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

We currently have no products that have been approved for commercial sale; however, the current and future use of our product candidates by us and our collaborators in clinical trials, expanded access programs, the sale of any

products in the future, 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 if we obtain marketing approval for any of our product candidates. 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.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur a liability and our research and development programs and the development of our product candidates could be delayed.

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 60% in a single day or decreased as much as 55% in a single day. We expect that our stock could have a material swing in its trading price in connection with the FDA’s upcoming decision relating to our eteplirsen NDA. 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 timing of our submissions to regulatory authorities and regulatory decisions and developments including any decision by the FDA regarding our NDA for eteplirsen;
- 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 our product candidates;
- delays in beginning and completing preclinical 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 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 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 such as the resignation of our former CEO and appointment of an interim CEO in 2015; 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 majority of the voting power of all the then-outstanding shares of voting stock;
- prohibition of cumulative voting of shares in the election of directors;
- right of the board of directors to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death, disqualification or removal of a director;
- express authorization of the board of directors to make, alter or repeal our bylaws;
- prohibition on stockholder action by written consent;
- advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at stockholder meetings;
- the ability of our board of directors to authorize the issuance of undesignated preferred stock, the terms and rights of which may be established and shares of which may be issued without stockholder approval, including rights superior to the rights of the holders of common stock; and
- a super-majority (66 2/3%) of the voting power of all of the then-outstanding shares of capital stock are required to amend, rescind, alter or repeal our bylaws and certain provisions of our certificate of incorporation.

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

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

Our quarterly operating results have fluctuated in the past, and we believe they will continue to do so in the future. Our operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Likewise, our research and development expenses may experience fluctuations as a result of the timing and magnitude

of expenditures incurred in

support of our DMD and other 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, 2016, there were approximately 45.8 million shares of common stock outstanding and outstanding awards to purchase 7.9 million shares of common stock under various incentive stock plans. Additionally, as of March 31, 2016, there were 0.6 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, less than 0.1 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and 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 5, 2016 By: /s/ EDWARD KAYE, MD
Edward Kaye, MD
Interim Chief Executive Officer, Senior Vice President, Chief Medical Officer

(Principal Executive Officer)

Date: May 5, 2016 By: /s/ SANDESH MAHATME
Sandesh Mahatme
Senior Vice President, Chief Financial Officer

(Principal Financial and Accounting Officer)

EXHIBIT INDEX

Exhibit		Incorporated by Reference to Filings Indicated				Provided
			File		Filing	
Number	Exhibit Description	Form	No.	Exhibit	Date	Herewith
31.1	Certification of the Company's Interim Chief Executive Officer, Edward Kaye, MD, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of the Company's Interim Chief Executive Officer, Edward Kaye, MD, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

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