Sarepta Therapeutics, Inc.
Form 10-Q
August 06, 2015
Table of Contents

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 June 30, 2015

OR

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

For the transition period from _____ to _____

Commission file number 001-14895

SAREPTA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

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

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

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes 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	**41,513,881**
(Class)	**(Outstanding as of July 31, 2015)**

Table of Contents

SAREPTA THERAPEUTICS, INC.

FORM 10-Q

INDEX

Table of Contents

PART I FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(unaudited, in thousands, except per share amounts)

	As of June 30, 2015	As of December 31, 2014
Assets		
Current Assets:		
Cash and cash equivalents	$ 69,538	$ 73,551
Short-term investments	87,349	136,793
Accounts receivable	2,416	2,416
Other current assets	23,091	35,036
Total Current Assets	182,394	247,796
Restricted cash and investments	783	782
Property and equipment, net of accumulated depreciation of $22,193 and $19,896 as of June 30, 2015 and December 31, 2014, respectively	37,196	38,501
Patent costs, net of accumulated amortization of $2,353 and $2,081 as of June 30, 2015 and December 31, 2014, respectively	6,024	5,891
Other assets	5,313	2,063
Total Assets	$ 231,710	$ 295,033
Liabilities and Stockholders Equity		
Current Liabilities:		
Accounts payable	$ 14,095	$ 12,408
Accrued expenses	15,184	17,366
Current portion of long-term debt	100	98
Current portion of notes payable	4,908	2,492
Deferred revenue	3,303	3,318
Other current liabilities	1,372	1,185
Total Current Liabilities	38,962	36,867
Long-term debt	20,652	1,476
Notes payable		2,262
Deferred rent and other	6,720	6,775

Total Liabilities	66,334	47,380
Commitments and contingencies		
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; 41,515,793 and 41,311,512 issued and outstanding at June 30, 2015 and December 31, 2014, respectively	4	4
Additional paid-in capital	947,826	926,769
Unrealized loss	(19)	(95)
Accumulated deficit	(782,435)	(679,025)
Total Stockholders Equity	165,376	247,653
Total Liabilities and Stockholders Equity	$ 231,710	$ 295,033

See accompanying notes to unaudited condensed consolidated financial statements.

Table of Contents

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited, in thousands, except per share amounts)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2015	2014	2015	2014
Revenue from research contracts and other grants	$	$ 2,583	$	$ 8,671
Operating expenses:				
Research and development	29,180	20,641	68,345	41,547
General and administrative	12,927	12,213	35,624	22,516
Total operating expenses	42,107	32,854	103,969	64,063
Operating loss	(42,107)	(30,271)	(103,969)	(55,392)
Other income (loss):				
Interest income and other, net	256	181	559	280
Loss on change in warrant valuation		(3,784)		(7,035)
Total other income (loss)	256	(3,603)	559	(6,755)
Net loss	$ (41,851)	$ (33,874)	$ (103,410)	$ (62,147)
Other comprehensive income (loss):				
Unrealized (loss) gain on short-term securities - available-for-sale	(2)	24	76	(35)
Total other comprehensive (loss) income	(2)	24	76	(35)
Comprehensive loss	$ (41,853)	$ (33,850)	$ (103,334)	$ (62,182)
Loss per share basic and diluted	$ (1.01)	$ (0.85)	$ (2.50)	$ (1.60)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	41,357	39,862	41,341	38,847

See accompanying notes to unaudited condensed consolidated financial statements.

4

Table of Contents

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited, in thousands)

	For the Six Months Ended June 30,	
	2015	**2014**
Cash flows from operating activities:		
Net loss	$ (103,410)	$ (62,147)
Adjustments to reconcile net income to cash flows in operating activities:		
Depreciation and amortization	2,571	1,483
Amortization of premium on available-for-sale securities and noncash interest	613	1,127
Loss on abandonment of patents	132	52
Stock-based compensation	20,086	9,929
Increase in warrant valuation		7,035
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable		(1,696)
Net decrease (increase) in other assets	8,695	(19,261)
Net increase in accounts payable, accrued expenses, deferred revenue and other liabilities	(566)	(4,291)
Net cash used in operations	(71,879)	(67,769)
Cash flows from investing activities:		
Release and maturity of restricted investments		3,250
Purchase of property and equipment	(1,169)	(9,841)
Patent costs	(640)	(628)
Purchase of available-for-sale securities	(49,631)	(226,616)
Maturity of available-for-sale securities	98,650	48,669
Net cash from (used in) investing activities	47,210	(185,166)
Cash flows from financing activities:		
Proceeds from borrowings, net of debt issuance costs	19,734	
Repayments of long-term debt	(49)	(47)
Proceeds from exercise of options and warrants and the sale of common stock, net of offering costs	971	98,771
Net cash from financing activities	20,656	98,724
Decrease in cash and cash equivalents	(4,013)	(154,211)
Cash and cash equivalents:		
Beginning of period	73,551	256,965

End of period	$	69,538	$	102,754
Supplemental disclosure of cash flow information:				
Cash paid during the period for interest	$	37	$	40
Supplemental schedule of non-cash investing activities and financing activities:				
Issuance of common stock in satisfaction of warrants and other liabilities	$		$	6,215
Tenant improvement paid by Landlord	$		$	65
Property and equipment included in accrued expenses	$		$	422
Accrual for the debt issuance costs related to the senior secured term loan	$	540	$	
Patent costs included in accrued expenses	$	170	$	200
Capitalized interest	$	99	$	

See accompanying notes to unaudited condensed consolidated financial statements.

Table of Contents

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. The Company is also developing therapeutics using its technology for the treatment of infectious diseases, such as drug resistant bacteria, 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 June 30, 2015, the Company had approximately $157.7 million of cash, cash equivalents and investments, consisting of $69.5 million of cash and cash equivalents, $87.3 million of short-term investments and $0.8 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments 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 contracts 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 on its own behalf or in collaboration with others. 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, 2014.

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, revenue recognition and income tax.

2. RECENT ACCOUNTING PRONOUNCEMENTS

In April 2015, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2015-03, which simplifies presentation of debt issuance costs. The amendments in this update require that debt issuance costs related to a recognized debt liability be presented in the balance sheet as a direct deduction from the carrying amount of that debt liability, consistent with debt discounts. ASU No. 2015-03 will be effective for fiscal years beginning after December 15, 2015, with early adoption permitted. The Company has elected to adopt this ASU early and the adoption of this guidance did not have a material effect on its consolidated financial statements. For additional information, please read *Note 7, Long-term Debt* of the unaudited condensed consolidated financial statements.

In August 2014, the FASB issued ASU No. 2014-15 which 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. Substantial doubt about an entity s ability to continue as a going concern exists when relevant conditions and events, considered in the aggregate, indicate that it is probable that the entity will not be able to meet its obligations as they become due within one year after the date that the financial statements are issued or available to be issued. If conditions or events raise substantial doubt about an entity s ability to continue as a going concern, but the

Table of Contents

substantial doubt is alleviated as a result of consideration of management s plans to mitigate those relevant conditions or events, the entity is required to disclose (1) principal conditions or events that raise substantial doubt about the entity s ability to continue as a going concern, (2) management s evaluation of the significance of those conditions or events in relation to the entity s ability to meet its obligations, and (3) management s plans that alleviate substantial doubt about the entity s ability to continue as a going concern. However, if conditions or events raise substantial doubt about an entity s ability to continue as a going concern, and substantial doubt is not alleviated after consideration of management s plans, an entity should include a statement in a footnote indicating that there is 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. ASU No. 2014-15 is effective for the annual period ending after December 15, 2016, with early adoption permitted. The Company has not adopted this guidance as of June 30, 2015, and based on the Company s financial condition as of June 30, 2015, 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, which amends the guidance for accounting for revenue from contracts with customers. This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, *Revenue Recognition,* and creates a new Topic 606, *Revenue from Contracts with Customers*. 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. This guidance is effective for the fiscal years beginning after December 15, 2016, with early adoption not permitted. In July 2015, the FASB decided to delay the effective date of this standard by one year, with early adoption only permitted in fiscal year 2017. Two adoption methods are permitted: retrospectively to all prior reporting periods presented, with certain practical expedients permitted; or retrospectively with the cumulative effect of initially adopting the ASU recognized at the date of initial application. 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 June 30, 2015			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 5,400	$ 5,400	$	$
Commercial paper	13,369		13,369	
Government and government agency bonds	47,952		47,952	
Corporate bonds	28,403		28,403	
Certificates of deposit	648	648		
Total assets	$ 95,772	$ 6,048	$ 89,724	$

	Fair Value Measurement as of December 31, 2014			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 47,740	$ 47,740	$	$
Commercial paper	2,997		2,997	
Government and government agency bonds	75,250		75,250	
Corporate bonds	58,546		58,546	
Certificates of deposit	647	647		
Total assets	$ 185,180	$ 48,387	$ 136,793	$

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 June 30, 2015.

Table of Contents

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 market observable 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 and notes payable 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 June 30, 2015 and December 31, 2014 was less than 3 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 June 30, 2015			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
		(in thousands)		
Cash and money market funds	$ 67,163	$	$	$ 67,163
Commercial paper	13,372		(3)	13,369
Government and government agency bonds	47,954		(2)	47,952
Corporate bonds	28,417		(14)	28,403
Total assets	$ 156,906	$	$ (19)	$ 156,887
As reported:				
Cash and cash equivalents	$ 69,538	$	$	$ 69,538
Short-term investments	87,368		(19)	87,349
Total assets	$ 156,906	$	$ (19)	$ 156,887

	As of December 31, 2014			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
		(in thousands)		
Cash and money market funds	$ 73,551	$	$	73,551
Commercial paper	2,997			2,997

Government and government agency bonds	75,289		(39)	75,250
Corporate bonds	58,602		(56)	58,546
Total assets	$ 210,439	$	$ (95)	210,344
As reported:				
Cash and cash equivalents	$ 73,551	$	$	73,551
Short-term investments	136,888		(95)	136,793
Total assets	$ 210,439	$	$ (95)	210,344

Table of Contents

5. OTHER CURRENT ASSETS

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

	As of June 30, 2015	As of December 31, 2014
	(in thousands)	
Manufacturing-related deposits	$ 19,550	$ 30,668
Prepaid expenses	3,086	2,797
Other	455	1,571
Total other current assets	$ 23,091	$ 35,036

6. ACCRUED EXPENSES

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

	As of June 30, 2015	As of December 31, 2014
	(in thousands)	
Accrued employee compensation costs	$ 4,955	$ 6,170
Accrued clinical and preclinical costs	4,845	3,471
Accrued professional fees	1,924	3,403
Accrued contract manufacturing costs	1,876	3,271
Accrued research costs	579	311
Accrued facility-related costs	270	300
Other	735	440
Total accrued expenses	$ 15,184	$ 17,366

7. LONG-TERM DEBT

On June 26, 2015, the Company entered into a credit and security agreement (the Credit Agreement) with MidCap Financial (Lender) that provides a senior secured term loan of $20.0 million. The principal amount may be increased by an additional $20.0 million, for an aggregate amount not to exceed $40.0 million, upon the acceptance by the Food and Drug Administration (FDA) of the new drug application (NDA) for eteplirsen. All obligations under the Credit Agreement are secured by substantially all of the Company s assets, excluding, without limitation, the Company s intellectual property, certain equity interests relating to foreign subsidiaries and all assets owned by foreign subsidiaries, among others.

Borrowings under the Credit Agreement bear interest at a rate per annum equal to 7.75%, with only interest payments due through June 30, 2016. In addition to paying interest on outstanding principal under the Credit Agreement, the

Company will pay an origination fee equal to 0.50% of the amount of the term loan when advanced under the Credit Agreement, as well as a final payment fee equal to 2.00% of the amount borrowed under the Credit Agreement when the term loan is fully repaid. Commencing on July 1, 2016 and continuing for the remaining twenty-four months of the facility, the Company will be required to make monthly principal payments of approximately $0.8 million, or monthly payments of approximately $1.7 million if the facility is increased by the additional $20.0 million referenced above.

The Company may voluntarily prepay outstanding loans under the Credit Agreement at any time, provided that the Company may not prepay an amount that is less than the total of all of the credit extensions and other related obligations under the Credit Agreement then outstanding. In the event of a voluntary prepayment, the Company is obligated to pay a prepayment fee equal to 2.95% of the outstanding principal of such advance if the prepayment is made within twelve months after the closing date, or 2.00% of the outstanding principal of such advance if the prepayment is made on or after the date that is twelve months after the closing date.

The Credit Agreement contains affirmative covenants that include government compliance, reporting requirements, maintaining property, making tax payments, maintaining insurance and cooperating during litigation. Additionally, the Company is required to maintain a minimum cash balance as collateral within its operating bank account with cash and cash equivalents of no less than the greater of the outstanding principal amount or $15.0 million. Negative covenants include restrictions on asset dispositions, acquisitions, indebtedness, liens, dividends and share purchases, amendments to material contracts and other restrictions.

The Credit Agreement includes customary events of default, including cross defaults, change of control and material adverse change. Additionally, the Company s failure to be compliant with the affirmative or negative covenants or make payments when they become due will result in an event of default.

Table of Contents

In connection with the senior secured term loan, the Company recorded $20.0 million as long-term debt in the unaudited condensed consolidated balance sheets as of June 30, 2015. In addition, the Company incurred approximately $0.8 million in debt issuance costs that were recorded as a direct deduction to the carrying value of the term loan in the unaudited condensed consolidated balance sheets. These costs are being amortized to interest expense using the effective interest method over the term of the loan. For the three and six months ended June 30, 2015, the Company recognized less than $0.1 million of interest expense related to the term loan.

The following table summarizes the components of the long-term debt recorded for the period indicated:

	As of June 30, 2015 (in thousands)
Principal amount	$ 20,000
Unamortized debt issuance expense	(773)
Net carrying value of senior secured term loan	19,227
Other long-term debt	1,525
Total long-term debt	$ 20,752

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 June 30,				For the Six Months Ended June 30,			
	2015		2014		2015		2014	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options*	367,167	$ 15.69	117,770	$ 23.68	1,974,711	$ 11.34	1,303,035	$ 19.80
Restricted stock awards	110,783	$ 13.54		$	116,783	$ 13.56	6,000	$ 29.03

* Majority of the stock options granted during the periods presented in the table have only service-based criteria and vest over four years.

Stock-based Compensation Expense

For the three months ended June 30, 2015 and 2014, total stock-based compensation expense was $5.9 million and $5.6 million, respectively. For the six months ended June 30, 2015 and 2014, total stock-based compensation expense

was $20.1 million and $9.9 million, respectively. Included in the amount for the six months ended June 30, 2015 is $8.6 million of stock-based compensation expense incurred in connection with the resignation of the Company s former Chief Executive Officer (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 June 30,		For the Six Months Ended June 30,	
	2015	2014	2015	2014
	(in thousands)			
Research and development	$ 2,562	$ 2,345	$ 5,008	$ 4,218
General and administrative	3,368	3,242	15,078	5,711
Total stock-based compensation expense	$ 5,930	$ 5,587	$ 20,086	$ 9,929

Table of Contents

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 June 30,		For the Six Months Ended June 30,	
	2015	2014	2015	2014
	(in thousands)			
Stock options	$ 5,099	$ 5,042	$ 18,650	$ 8,953
Restricted stock awards	132	72	174	136
Restricted stock units		5		1
Stock appreciation rights	115	146	262	293
Employee stock purchase plan	584	322	1,000	546
Total stock-based compensation expense	$ 5,930	$ 5,587	$ 20,086	$ 9,929

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, they are excluded from the diluted net loss per share calculation.

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2015	2014	2015	2014
	(in thousands, except per share amounts)			
Net loss	$ (41,851)	$ (33,874)	$ (103,410)	$ (62,147)
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	41,357	39,862	41,341	38,847
Dilutive effect of outstanding warrants, 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	41,357	39,862	41,341	38,847
Net loss per share basic and diluted	$ (1.01)	$ (0.85)	$ (2.50)	$ (1.60)

*

For the three and six months ended June 30, 2015, stock options, restricted stock awards and stock appreciation rights to purchase approximately 7.0 million shares of common stock were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

For the three and six months ended June 30, 2014, warrants, stock options, restricted stock awards, restricted stock units and stock appreciation rights to purchase approximately 5.9 million shares of common stock were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

10. COMMITMENTS AND CONTINGENCIES

Purchase Commitments

The following table presents non-cancelable contractual obligations arising from arrangements that the Company has entered into from time to time for the provision of goods and services:

	As of June 30, 2015 (in thousands)
2015 (6 months)	$ 27,236
2016	41,312
2017	23,940
2018	14,260
2019	5,705
Total purchase commitments	$ 112,453

Table of Contents

Litigation

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (*Corban v. Sarepta, et. al., No. 14-cv-10201*) by order of the court on June 23, 2014, 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 seeks damages in an unspecified amount. Pursuant to the court s June 23, 2014 order, Sarepta filed a motion to dismiss the consolidated amended complaint on August 18, 2014, and argument on the motion was held on March 12, 2015. On March 31, 2015, the Court dismissed plaintiffs amended complaint. Plaintiffs in the Corban suit have filed a motion for leave seeking to file a further amended complaint, which the Company has opposed. A hearing on plaintiffs motion for leave is scheduled for August 12, 2015.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 by William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Christopher Garabedian, and Sandesh Mahatme (*Kader v. Sarepta et.al 1:14-cv-14318*), asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, Christopher Garabedian and Sandesh Mahatme. Plaintiffs amended complaint, filed on March 20, 2015, alleges 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. Plaintiffs seek compensatory damages and fees. The Company received service of the complaint on January 5, 2015. Sarepta filed a motion to dismiss the complaint on May 4, 2015, pursuant to the scheduling order entered on February 20, 2015, which plaintiffs have opposed. Oral argument on the motion to dismiss has not been scheduled.

In addition, two 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*, *Cause 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 26, 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.

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 should have a material financial impact on the Company.

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, 2014 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

Table of Contents

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, potential, likely, seek 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 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 additional data and analysis collected by us on eteplirsen;*

- *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 of the FDA s interpretations on our FDA submissions (including our investigational new drug applications and new drug applications (NDAs)), filing decisions by the FDA, advisory committee recommendations, and FDA product approval decisions and related timelines;*

- *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 current and planned investment 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 for pre-launch and commercial positions and the impact of winding down or terminating these commitments if the FDA does not file or approve our eteplirsen NDA;*

- *our ability to raise additional funds to support our business plans and the impact of our credit and security agreement with MidCap 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 that depends on our ability to satisfactorily and timely respond to recent FDA requests with respect to preclinical data on the exon 53 skipping product candidate;

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 (API s) 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 and any issuance of an Emergency Use Authorization (EUA) for our product candidates intended to treat Ebola virus and Marburg virus, 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;

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;

Table of Contents

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;

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 (USPTO) 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 Prosensa Holding N.V. (Prosensa), which is now owned 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 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;

our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements;

our succession plan, including the search for a permanent full-time 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; and

other factors set forth below under the heading Risk Factors .

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 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, the fact that: the FDA may not file our NDA submission for eteplirsen or 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 submission; the additional information and data we collect for eteplirsen may not be consistent with prior data or results or may not support our eteplirsen NDA submission, a filing of the NDA, a positive advisory committee vote, if any, or approval of eteplirsen; 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; 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

Table of Contents

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 or business plan; we may not be able to attract sufficient capital or to enter into strategic relationships; the outcome of 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. We filed an NDA for eteplirsen for the treatment of DMD at the end of June 2015. If we are successful in our development efforts, eteplirsen will address a severe but unmet medical need. We are in the process of conducting or initiating several studies for product candidates designed to skip exons 45, 51 and 53 in the U.S. and the European Union (E.U.). These include an ongoing open label extension study following completion of our initial Phase IIb clinical trials, several clinical trials in exon 51 amenable genotypes, including a confirmatory study in ambulatory patients, studies on participants with early stage and advanced stage DMD, a dose-ranging study for our product candidate designed to skip exon 45 and a placebo-controlled confirmatory study with product candidates designed to skip exons 45 and 53. Human dosing with the exon 53 skipping product candidate cannot begin in the U.S. in our placebo-controlled confirmatory study until we satisfactorily respond to recent FDA requests for additional safety information and analyses on preclinical study data. In particular, the FDA s requests include clarification on the potential cause(s) of certain adverse events in rats at the highest dose used in preclinical studies with the exon 53 skipping product candidate and analyses on the potential applicability and addressability in humans. Although we are working to respond quickly to these FDA requests, it is not determinable at this time what

delays, if any, this will have on the timeline or trial design for our placebo-controlled confirmatory study. We have successfully completed Part 1, a dose escalation phase in humans, of a Phase I/IIa clinical trial for this exon 53 skipping product candidate in the E.U. with the SKIP-NMD Consortium. As announced by the SKIP-NMD consortium in July 2015, after no significant problems were identified in humans during the dose escalation portion of the study with this exon 53 skipping product candidate, the E.U. data safety monitoring board gave its approval to proceed with Part 2 of the SKIP-NMD study, which includes treated and untreated arms. We have also leveraged the capabilities of our RNA-targeted technology platforms to develop therapeutics for the treatment of infectious diseases such as influenza, Marburg and Ebola under prior contracts with the Department of Defense (DoD), however, further development of these product candidates would be conditioned, in part, on obtaining additional funding or collaborations. Our discovery and research programs include collaborations with various third parties and focus on developing therapeutics in rare, genetic, anti-infective, neuromuscular and central nervous system diseases among other diseases. We are exploring the application of our proprietary PMO platform technology and toll-like receptor technology in various diseases including drug resistant bacteria, DMD, Becker muscular dystrophy, progeria, adult onset Pompe disease, lupus and graft-versus-host disease.

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®, PMO*plus*® and PPMO. PMOs 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. PMOs have demonstrated inhibition of messenger RNA (mRNA) translation and alteration of pre-mRNA splicing. The chemistry of PMO-based molecules has 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, PMOs 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.

Table of Contents

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 do achieve revenue from product sales, we are likely to continue to incur operating losses in the near term.

As of June 30, 2015, we had approximately $157.7 million of cash, cash equivalents and investments, consisting of $69.5 million of cash and cash equivalents, $87.3 million of short-term investments and $0.8 million of restricted cash and investments. We believe that the balance of our cash, cash equivalents and investments is sufficient to fund our current operational plan for the next twelve months. We may pursue additional cash resources through public or private financings, establish credit or debt facilities or collaborations with, or license our technology to, other companies. For example, on June 29, 2015, we announced that we had entered into a credit and security agreement that provides a senior secured term loan of $20.0 million (which may be increased by an additional $20.0 million, for an aggregate amount not to exceed $40.0 million, upon the acceptance by the FDA of our NDA for eteplirsen).

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 our industry and the complex regulatory environment in which we operate. There can be no assurance that we will ever achieve significant revenue or profitable operations.

Key Financial Metrics

Revenue

Government Research Contract and Grant Revenue. In the periods presented in this report, substantially all of our revenue was derived from our former research and development contracts with and grants from the U.S. government. As of December 31, 2014, we had completed all development activities under our contracts with the U.S. government. We recognize revenue from U.S. government research contracts and 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.

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 that is separate and independent of our performance under the other elements of the arrangement. 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 June 30, 2015, we had deferred revenue of $3.3 million, which represents up-front fees which we will 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 have completed Phase I and plan to conduct Phase II of a Phase I/IIa clinical trial for an exon 53 skipping product candidate in the E.U. and are initiating a dose-ranging study for our exon 45 skipping product candidate in the U.S. We also plan to include our exon 45 and the exon 53 skipping product candidates in our planned placebo-controlled confirmatory clinical trial in the U.S. and the E.U. The remainder of our research and development programs are in various stages of research and pre-clinical development. However, our research and development efforts may not result in any approved products. Product candidates that appear promising at early stages of development may not reach the market for a variety of reasons. Similarly, any of our product candidates may be found to be unsafe or ineffective during clinical trials, may have clinical trials that take longer to complete than anticipated, may fail to receive necessary regulatory approvals, or may prove impracticable to manufacture in commercial quantities at reasonable cost and with acceptable quality.

As a result of these uncertainties and 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.

Table of Contents

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 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 Income and Other, Net. Interest income and other, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense and rental income and loss. Our cash equivalents and investments consist of commercial paper, government and government agency debt securities, money market investments and certificates of deposit. Interest expense includes interest accrued on our promissory note related to the Andover, Massachusetts facility, our senior secured term loan and our mortgage loan related to our Corvallis, Oregon property, a substantial portion of which has been leased to a third party since November 2011. Rental income and loss is from leasing excess space in some of our facilities.

Loss on Change in Warrant Valuation. Warrants issued in connection with our January and August 2009 financings were classified as liabilities as opposed to equity due to their settlement terms. These warrants were non-cash liabilities and we were not required to expend any cash to settle these liabilities. The fair value of these warrants was recorded on our unaudited condensed consolidated balance sheets at the date of issuance and the warrants were marked to market at each financial reporting period, with changes in the fair value recorded as Loss on change in warrant valuation in our unaudited condensed consolidated statements of operations and comprehensive loss. The fair value of the warrants was determined using the Black-Scholes-Merton option-pricing model, which required the use of significant judgment and estimates related to the inputs used in the model. All warrants issued in January and August 2009 were exercised or expired during 2014.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations are 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 tax.

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, 2014 filed with the SEC on February 26, 2015.

Table of Contents

Results of Operations for the Three and Six Months Ended June 30, 2015 and 2014

The following table sets forth selected consolidated statements of operations data for each of the periods indicated:

	For the Three Months Ended June 30,			
	2015	2014	Change	Change
	(in thousands, except per share amounts)		$	%
Revenue from research contracts and other grants	$	$ 2,583	$ (2,583)	-100%
Operating expenses:				
Research and development	29,180	20,641	8,539	41%
General and administrative	12,927	12,213	714	6%
Total operating expenses	42,107	32,854	9,253	28%
Operating loss	(42,107)	(30,271)	(11,836)	39%
Other income (loss):				
Interest income and other, net	256	181	75	41%
Loss on change in warrant valuation		(3,784)	3,784	-100%
Net loss	$ (41,851)	$ (33,874)	$ (7,977)	24%
Loss per share basic and diluted	$ (1.01)	$ (0.85)	$ (0.16)	19%

	For the Six Months Ended June 30,			
	2015	2014	Change	Change
	(in thousands, except per share amounts)		$	%
Revenue from research contracts and other grants	$	$ 8,671	$ (8,671)	-100%
Operating expenses:				
Research and development	68,345	41,547	26,798	65%
General and administrative	35,624	22,516	13,108	58%
Total operating expenses	103,969	64,063	39,906	62%
Operating loss	(103,969)	(55,392)	(48,577)	88%
Other income (loss):				
Interest income and other, net	559	280	279	100%
Loss on change in warrant valuation		(7,035)	7,035	-100%

Net loss		$ (103,410)	$ (62,147)	$ (41,263)	66%
Loss per share	basic and diluted	$ (2.50)	$ (1.60)	$ (0.90)	56%

Revenue

As of December 31, 2014, we had completed all development activities of our contracts with the U.S. government. Therefore, no revenue was recognized for the three or six months ended June 30, 2015. For the three and six months ended June 30, 2014, we recognized $2.6 million and $8.7 million, respectively, under our U.S. government contracts. The majority of the revenue under our U.S. government contracts has been recognized as of June 30, 2015 and only revenue for contract finalization, if any, is expected in the future.

Table of Contents

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 table summarizes research and development expenses by project for each of the periods indicated:

	For the Three Months Ended June 30,			
	2015	2014	Change $	Change %
	(in thousands)			
Eteplirsen (exon 51)	$ 11,858	$ 6,462	$ 5,396	84%
Exon 53	1,243	371	872	235%
Exon 45	1,065	785	280	36%
Other projects	453	1,862	(1,409)	-76%
Internal research and development expenses	14,561	11,161	3,400	30%
Total research and development expenses	$ 29,180	$ 20,641	$ 8,539	41%

	For the Six Months Ended June 30,			
	2015	2014	Change $	Change %
	(in thousands)			
Eteplirsen (exon 51)	$ 33,500	$ 12,360	$ 21,140	171%
Exon 45	3,949	1,072	2,877	268%
Exon 53	2,170	2,856	(686)	-24%
Other projects	1,045	4,064	(3,019)	-74%
Internal research and development expenses	27,681	21,195	6,486	31%
Total research and development expenses	$ 68,345	$ 41,547	$ 26,798	65%

The following table summarizes research and development expenses by category for each of the periods indicated:

	For the Three Months Ended June 30,			
	2015	2014	Change $	Change %
	(in thousands)			
Clinical and manufacturing expenses	$ 12,822	$ 6,676	$ 6,146	92%
Compensation and other personnel expenses	6,142	5,049	1,093	22%

Stock-based compensation	2,562	2,345	217	9%
Professional services	2,713	2,559	154	6%
Facility-related expenses	2,529	1,777	752	42%
Preclinical expenses	1,162	1,205	(43)	-4%
Research and other	1,250	1,030	220	21%
Total research and development expenses	$ 29,180	$ 20,641	$ 8,539	41%

	For the Six Months Ended June 30,			
	2015	2014	Change $	Change %
	(in thousands)			
Clinical and manufacturing expenses	$ 36,676	$ 15,246	$ 21,430	141%
Compensation and other personnel expenses	12,137	9,731	2,406	25%
Stock-based compensation	5,008	4,218	790	19%
Facility-related expenses	4,908	3,137	1,771	56%
Professional services	3,821	3,908	(87)	-2%
Preclinical expenses	2,508	2,095	413	20%
Research and other	3,287	3,212	75	2%
Total research and development expenses	$ 68,345	$ 41,547	$ 26,798	65%

Table of Contents

Research and development expenses for the three months ended June 30, 2015 increased by $8.5 million, or 41%, compared with the three months ended June 30, 2014. The increase was primarily due to increases of $6.1 million in clinical and manufacturing expenses, driven by increased enrollment in our ongoing clinical trials and timing of manufacturing activities, including raw material purchases, $1.3 million in compensation expenses, primarily driven by increases in headcount and $0.8 million in facility-related expenses, primarily driven by corporate growth.

Research and development expenses for the six months ended June 30, 2015 increased by $26.8 million, or 65%, compared with the six months ended June 30, 2014. The increase was primarily due to increases of $21.4 million in clinical and manufacturing expenses driven by increased enrollment in our ongoing clinical trials and timing of manufacturing activities, including raw material purchases, $3.2 million in compensation expenses primarily driven by increases in headcount, and $1.8 million in facility-related expenses, primarily driven by corporate growth.

General and Administrative Expenses

The following table summarizes general and administrative expenses by category for each of the periods indicated:

	For the Three Months Ended June 30,			
	2015	2014	Change $	Change %
	(in thousands)			
Professional services	4,385	4,284	101	2%
Stock-based compensation	3,473	3,242	231	7%
Compensation and other personnel expenses	3,431	3,051	380	12%
Other	1,638	1,636	2	0%
Total general and administrative expenses	$ 12,927	$ 12,213	$ 714	6%

	For the Six Months Ended June 30,			
	2015	2014	Change $	Change %
	(in thousands)			
Former CEO severance expense	$ 9,182	$	$ 9,182	NA
Professional services	8,254	6,776	1,478	22%
Compensation and other personnel expenses	7,129	6,055	1,074	18%
Stock-based compensation	6,520	5,711	809	14%
Other	4,539	3,974	565	14%
Total general and administrative expenses	$ 35,624	$ 22,516	$ 13,108	58%

General and administrative expenses for the three months ended June 30, 2015 increased by $0.7 million, or 6%, compared with the three months ended June 30, 2014. This was primarily due to increases in headcount.

General and administrative expenses for the six months ended June 30, 2015 increased by $13.1 million, or 58%, compared with the six months ended June 30, 2014. The increase was primarily due to a $9.2 million severance

expense, including stock-based compensation, as a result of the resignation of our former CEO, and increases of $1.9 million in compensation expenses, primarily driven by increases in headcount, and $1.5 million in professional services due to increased legal fees.

Interest Income and Other, Net

Interest income and other, net, for the three months ended June 30, 2015 increased by $0.1 million compared with the three months ended June 30, 2014. The increase was primarily driven by rental income from the leasing of unused office space.

Interest income and other, net, for the six months ended June 30, 2015 increased by $0.3 million compared with the six months ended June 30, 2014. The increase was primarily driven by rental income from the leasing of unused office space.

Loss on Change in Warrant Valuation

All warrants issued in January and August 2009 were exercised or expired during 2014 and we no longer have outstanding warrants. Therefore, we did not have any change in warrant valuation for the three or six months ended June 30, 2015. For the three and six months ended June 30, 2014, we had an approximately $3.8 million and $7.0 million loss on change in warrant valuation, respectively.

Table of Contents

Liquidity and Capital Resources

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

	As of June 30, 2015	As of December 31, 2014	Change $	Change %
	(in thousands)			
Financial assets:				
Cash and cash equivalents	$ 69,538	$ 73,551	$ (4,013)	-5%
Short-term investments	87,349	136,793	(49,444)	-36%
Restricted cash and investments	783	782	1	0%
Total cash, cash equivalents and investments	$ 157,670	$ 211,126	$ (53,456)	-25%
Borrowings:				
Long-term debt	$ 20,752	$ 1,574	$ 19,178	1218%
Notes payable	4,908	4,754	154	3%
Total borrowings	$ 25,660	$ 6,328	$ 19,332	305%
Working capital				
Current assets	$ 182,394	$ 247,796	$ (65,402)	-26%
Current liabilities	38,962	36,867	2,095	6%
Total working capital	$ 143,432	$ 210,929	$ (67,497)	-32%

For the period ended June 30, 2015, our principal sources of liquidity were from both equity and debt financing. For the period ended June 30, 2014, our principal source of liquidity was primarily equity financing. 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 Six Months Ended June 30,			
	2015	2014	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (71,879)	$ (67,769)	$ (4,110)	6%
Investing activities	47,210	(185,166)	232,376	-125%
Financing activities	20,656	98,724	(78,068)	-79%
Decrease in cash and cash equivalents	$ (4,013)	$ (154,211)	$ 150,198	-97%

Operating Activities. The increase in the amount of cash used in operating activities of $4.1 million for the six months ended June 30, 2015 compared with the six months ended June 30, 2014 was primarily due to an increase of $41.3 million in net loss driven by increases in research and development and general and administrative expenses offset by a favorable change of $33.4 million in operating assets and liabilities due to the timing of certain activities and an increase in non-cash adjustments of $3.8 million.

Table of Contents

Investing Activities. Cash provided by investing activities was $47.2 million for the six months ended June 30, 2015. Cash used in investing activities was $185.2 million for the six months ended June 30, 2014. The change was primarily due to an increase of $50.0 million from maturity of available-for-sale securities and decreases of $177.0 million from the purchase of available-for-sale securities, as well as $8.7 million from the purchase of property and equipment, partially offset by maturity of restricted investment of $3.3 million for the six months ended June 30, 2014.

Financing Activities. The decrease in the amount cash from financing activities of $78.1 million for the six months ended June 30, 2015 compared to the six months ended June 30, 2014 was primarily due to a decrease of $97.8 million in net proceeds from the issuance of common stock and warrant and stock option exercises, partially offset by an increase of $19.7 million in net proceeds from the senior secured term loan. For additional information, please read *Note 7, Long-term Debt* of the unaudited condensed consolidated financial statements contained in Part I, Item 1 of this report, Form 10-Q for the quarterly period ended June 30, 2015.

Contractual Obligations and Contingencies

In our continuing operations, we have entered into long-term contractual arrangements for our facilities and the provision of goods and services. The following table presents non-cancelable contractual obligations arising from these arrangements as of June 30, 2015:

		Payment Due by Period			
	Total	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
	(in thousands)				
Long-term debt (1)	$ 25,096	$ 1,635	$ 21,981	$ 343	$ 1,137
Notes payable (1)	5,019	5,019			
Lease obligations	27,018	4,542	9,458	9,933	3,085
Purchase obligations (2)	112,453	48,199	51,420	12,834	
Total contractual obligations and contingencies	$ 169,586	$ 59,395	$ 82,859	$ 23,110	$ 4,222

(1) Interest is included.

(2) Purchase obligations include agreements to purchase goods or services that are enforceable and legally binding or subject to cancellation fees. Purchase obligations relate primarily to our DMD development programs.

Milestone Obligations

We are obligated to make up to $36.0 million of future development 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 June 30, 2015, 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 June 30, 2015.

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 June 30, 2015, we had $157.7 million of cash, cash equivalents and investments, comprised of $69.5 million of cash and cash equivalents, $87.3 million of short-term investments and $0.8 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 June 30, 2015, 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.

Table of Contents

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 report, Form 10-Q for the quarterly period ended June 30, 2015, 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 June 30, 2015, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the six months ended June 30, 2015, 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*) 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 seeks damages in an unspecified amount. Pursuant to the court s June 23, 2014 order, Sarepta filed a motion to dismiss the consolidated amended complaint on August 18, 2014, and argument on the motion was held on March 12, 2015. On March 31, 2015, the Court dismissed plaintiffs amended complaint. Plaintiffs in the Corban suit have filed a motion for leave seeking to file a further amended complaint, which the Company has opposed. A hearing on plaintiffs motion for leave is scheduled for August 12, 2015.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 by William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Christopher Garabedian, and Sandesh Mahatme (*Kader v. Sarepta et.al 1:14-cv-14318*), asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, Christopher Garabedian and Sandesh Mahatme. Plaintiffs amended complaint, filed on March 20, 2015, alleges 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. Plaintiffs seek compensatory damages and fees. The Company received service of the complaint on January 5, 2015. Sarepta filed a motion to dismiss the complaint on May 4, 2015, pursuant to the scheduling order entered on February 20, 2015, which plaintiffs have opposed. Oral argument on the motion to dismiss has not been scheduled.

In addition, two 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*, *Cause 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 26, 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

Table of Contents

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.

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 should 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 Relating to Our Business

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

With the exception of eteplirsen, which is being studied in several studies, including a confirmatory clinical trial, and the exon 53 skipping product candidate for which we have completed a phase I trial and plan to conduct a phase II clinical trial in the E.U. with the Skip-NMD consortium, our product candidates are in relatively 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 in DMD, the exon 53 skipping product candidate we are developing with the SKIP-NMD consortium, and AVI-7100 in influenza are in active clinical development. We have an open IND for our exon 45 skipping product candidate for which we plan to begin a dose-ranging study later this year and plan to begin a clinical trial in the U.S. and the E.U. for exon 45 and 53 skipping product candidates. AVI-7537 in Ebola and AVI-7288 in Marburg were being developed through a program with the DoD and further development is conditioned in part on obtaining additional funding, collaborations or emergency use. Our other product candidates are in preclinical development or inactive. 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.

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

Our RNA-targeted platforms, utilizing proprietary PMO-based technology, have not been incorporated into a therapeutic commercial product and are still at a relatively early stage of development. This technology is used in all of our product candidates, including eteplirsen. Although we have conducted clinical studies with eteplirsen 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 position.

Table of Contents

We have been granted orphan 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 be able to be granted orphan product status at the time of 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 United States, meaning the FDA will generally 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. Although we may have drug 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 European Union, 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 European Union 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 before we do of an orphan drug application in the United States or the European Union for a product candidate that has the same active ingredient or is a similar medicinal product for the same indication as any of our drug candidates for which we plan to file for orphan designation and status. If that were to happen, our orphan drug applications 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 European Union, as applicable. Moreover, we cannot guarantee that another company will not receive advanced approval to market a product candidate that is granted orphan drug status in the United States or the European Union for a product candidate that has the same active ingredient or is a similar medicinal product for the same indication as any of our drug candidates for which we plan to file a new drug application or marketing authorization application. If that were to happen, any pending new drug application or marketing authorization application 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 European Union, 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 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 and the medical community which could limit commercialization of the product. Assuming that any of our product candidates receives the required regulatory approvals, commercial success will depend on a number of factors, including but not limited to the following:

- 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;

Table of Contents

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

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 (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 or an advisory committee could disagree with our beliefs, interpretations and conclusions regarding data we submit in connection with an NDA submission, including any of the additional information and data we collect for the eteplirsen NDA, or other product candidates, and may delay, reject or refuse to file our NDA submission until we meet their additional requirements, if ever. Even if we meet such requirements and our NDA is accepted for review or filed, the FDA could still deny approval of our product candidates, including eteplirsen, based on their review of the data or other factors.

The regulatory approval process for product candidates targeting orphan diseases, such as DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, and novel endpoints, such as dystrophin measures, and natural history data, is uncertain due to 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. For example, it is unclear how the FDA will interpret and implement FDASIA provisions, in particular, in considering what the appropriate regulatory approval pathway is for eteplirsen. We cannot be sure that any of our drug candidates will qualify for any of these 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 analysis), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, even if initially accepted, regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could result in a decision by the Company to not proceed with development of a product candidate including a decision to not submit an NDA for a product based on feedback from regulators. Furthermore, we have or are currently in the process of collecting additional eteplirsen data and analysis, some in direct response to FDA requests. For example, the FDA has previously expressed concerns with dystrophin as a surrogate endpoint and has requested an independent assessment of dystrophin positive fibers measured in our eteplirsen Phase IIb study. The FDA has also requested matched natural history to better evaluate the ongoing clinical results of our eteplirsen 201/202 study. Any material inconsistencies between our existing data and analysis and the new and additional data we have or are collecting, including the independent assessment of dystrophin positive fibers, safety data, matched natural history and data from a fourth biopsy, could negatively impact our eteplirsen NDA submission. Additionally, the FDA may determine, after evaluating the totality of our data and analysis package or receiving the vote of an advisory committee, if one is convened, that such package does not support an NDA filing or 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. For example, we recently received FDA requests relating to safety data at the highest dose in preclinical studies with an exon 53 skipping product candidate. 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 skipping product candidate. 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 our NDA submissions.

Table of Contents

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 could delay or eliminate any potential commercialization or product revenue for us. 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. 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, 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 met their primary endpoints of dystrophin production based on the measurements taken at weeks 24 and 48, respectively, and six-minute walk test 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. For example, on July 10, 2014, we announced the results for week 144 in Study 202, which showed a decline in walking ability at a rate slower than would be expected based on available DMD natural history; however, the decline on the six-minute walk test from baseline, although in prior study results was below 5%, was measured at 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 all patients showed a decline in distance walked on this measure since the week 144 time point. If the data from the confirmatory studies for eteplirsen do not produce the safety and efficacy data required by regulatory authorities for an NDA submission, filing, positive advisory committee vote or approval, 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. 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 and the 168 week clinical data from Study 202. Additionally, the guidance requested more specific data, such as a minimum duration of safety in new patients exposed to eteplirsen, patient-level natural history data to be obtained by us from independent academic institutions and MRI data from a recent study conducted

by an independent group. The FDA also indicated that further discussion would be needed to determine what would constitute a complete NDA submission. Additionally, if the results of the additional data we collect and provide in response to the FDA s request may not be consistent with prior results or may not support our NDA filing, a positive advisory committee vote, if any, or the approval of our eteplirsen NDA submission.

Table of Contents

We currently rely on third parties in the manufacturing process to produce our product candidates and our dependence on these parties, or our inability to engage third parties to meet manufacturing needs for large-scale clinical trials or potential commercial needs within sufficient timelines, may impair the advancement of our research and development programs and potential commercialization of our product candidates.

We do not currently 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. We therefore rely on, and expect for the foreseeable future to continue relying on, 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.

We have not engaged or contracted with all the third parties needed for the production of materials and APIs for any of our product candidates, including eteplirsen, in quantities sufficient for their potential commercial demand or for multiple large-scale clinical trials. In light of the limited number of third parties with the expertise to produce our product candidates, and the 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. 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 standards.

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 standards. We do not have control over a third-party manufacturer s compliance with these regulations and standards. In addition, changes in cGMP standards 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 standards, 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 standards 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.

To date, our product candidates have been manufactured in small quantities for preclinical studies and early stage clinical trials. As we prepare for larger and later stage clinical trials for our product candidates, including eteplirsen, and potential commercialization, we are working to increase the manufacturing capacity and scale up production of some of the components of our drug products. During 2015, we will continue to increase material and API production capacity to provide the drug product needed for additional eteplirsen trials and studies for our other product candidates (including a placebo-controlled study planned for one or more of our follow-on exon product candidates) and any planned subsequent commercialization, on an accelerated or other pathway. We may not be able to

Table of Contents

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 standards 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 could prevent commercialization of our product candidates.

We are currently winding down our expired U.S. government contract and further development of 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 and other government requirements successfully, 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;

Table of Contents

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:

Negotiating contracts and other related documents with clinical trial parties and Institutional Review Boards (IRBs), such as informed consents, CRO agreements and site agreements, 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 $42.1 million for three months ended June 30, 2015. Our accumulated deficit was $782.4 million as of June 30, 2015. 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 material 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;

acquire or in-license other product candidates;

initiate additional clinical trials for our product candidates;

seek marketing approvals for our product candidates that successfully complete clinical trials;

ultimately 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 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

Table of Contents

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.

If the FDA does not file or approve our eteplirsen NDA, our business may be negatively impacted and we may suffer financial losses in connection with winding down and terminating contracts, manufacturing commitments and employees hired in connection with our current and planned activities in preparation for a potential commercial launch.

Given the potential commercialization timelines, we have commenced certain pre-launch and commercialization investments and activities including 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 file or provide approval for our eteplirsen NDA, or we need to delay or discontinue our development and commercialization plans for eterplirsen for other reasons, our business may be negatively impacted and we may incur financial losses in connection with winding down our or terminating the investments, contracts and commitments we enter into for the purpose of positioning ourselves for a commercial launch of eteplirsen.

We will need additional funds to conduct our planned research, development and manufacturing efforts. If we fail to attract significant capital 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 new patent rights, regulatory changes, competitive and technological developments in the market and future 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 does not file the NDA for eteplirsen we submitted earlier this year, raising additional funds may be difficult or challenging. 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 April 29, 2014, we sold 2,650,000 shares of our common stock in an underwritten public offering at a price to the public of $38.00 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 new drug application 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.

Table of Contents

Any of these factors could materially and adversely affect our business, financial condition and results of operations. 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 CEO and President resigned on March 31, 2015 and we have appointed an interim CEO. No assurance can be made about the impact that this change in management will have on the Company and its business plans (including our regulatory and clinical plans and relationships) nor 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.

Table of Contents

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.

For example, although there was no material error in our consolidated financial statements, in connection with our assessment of the effectiveness of internal control over financial reporting as of December 31, 2013, our management identified a material weakness in our internal control over financial reporting. We designed and implemented controls to address the material weakness that was identified. However, we cannot provide assurances that material weaknesses in our internal control over financial reporting will not be identified in the future. Any failure to maintain or implement new or improved internal controls, or any difficulties that we may encounter in their maintenance or implementation, could result in additional material weaknesses or material misstatements in our consolidated financial statements and cause us to fail to meet our reporting obligations or prevent fraud, which could cause the trading price of our common stock to decline. We may not be able to build the human resources and infrastructure necessary to support the growth of our business or to appropriately implement our compliance controls and procedures. The time and resources required to build up our human resources and implement systems and infrastructure that may be needed to support our growth and compliance with applicable rules and regulations is uncertain, and failure to complete these 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 rights under European patents and patent applications. 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 a third party.

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 might not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. For example, in July 2014, the Patent Trial and Appeal Board (the PTAB) of the USPTO

Table of Contents

declared patent interferences between certain patents held by Sarepta (under license from the University of Western Australia, UWA) and patent applications held by Prosensa (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 Prosensa 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 Prosensa 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 Prosensa 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 Prosensa s/AZL s U.S. Application No. 14/248,279. If final resolution of the interferences and related appeals, if any, are not in our favor, then the Sarepta/UWA patents and any other Sarepta patents or applications also found to be interfering may be invalidated, 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 and related appeals, if any, are not in our favor, then the USPTO may issue the Prosensa/AZL patent applications resulting in the grant of one or more patents that may provide a basis for Prosensa to allege that our drug candidates, eteplirsen and/or SRP-4053, infringe such patents. These interferences 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, Prosensa 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 have been issued to Prosensa in Japan and certain other countries outside of Europe and the United States that may provide the basis for Prosensa or other parties to assert that eteplirsen infringes on such claims. Because we have not yet initiated an invalidation proceeding in Japan, 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.

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.

Table of Contents

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. Prosensa, which is developing competitive pipeline products, has rights to patent claims that, absent a license, may preclude us from commercializing eteplirsen in several jurisdictions. Prosensa 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 Europe would infringe on such patent. Both we and Prosensa have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other s briefs. Prosensa 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 effect on our business and leaves open the possibility that Prosensa or other parties that have rights to such patent could assert that our drug candidate, eteplirsen, infringes on such patent. The timing and outcome of appeal cannot be predicted or determined as of the date of this report. If as part of any appeal in the European Union we are unsuccessful in invalidating Prosensa 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 for our pan-exon strategy could be materially impaired.

We are also aware of existing patent claims Prosensa 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 Prosensa or other parties to assert that commercialization of eteplirsen and certain other of our product candidates would infringe on such claims.

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 others 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., 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 Pharmaceuticals (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.

We may face competitive risks arising from the Prosensa exon skipping platform and product candidate pipeline, which may include limitations on our ability to gain market share in the DMD space or other diseases targeted by our exon skipping platform and product candidate pipeline. For example, BioMarin Pharmaceuticals completed its rolling NDA submission for and announced the FDA s filing decision of the NDA submission for drisapersen, an exon 51 skipping product candidate that directly competes with eteplirsen, our lead product candidate.

Table of Contents

Other potential competitors include large, fully integrated pharmaceutical companies and more established biotechnology companies that have significantly greater resources and expertise in research and development, manufacturing, testing, obtaining regulatory approvals and marketing. Also, academic institutions, government agencies and other public and private research organizations conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and marketing. It is possible that these competitors will succeed in developing technologies that are more effective than our product candidates or that would render our technology obsolete or noncompetitive. Our competitors may, among other things:

develop safer or more effective products;

implement more effective approaches to sales and marketing;

develop less costly products;

obtain regulatory approval more quickly;

have access to more manufacturing capacity;

develop products that are more convenient and easier to administer;

form more advantageous strategic alliances; or

establish superior intellectual property positions.

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

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

Table of Contents

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 been historically volatile. Historically, 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, in a single day, our stock has increased as much as 60% in a single day or decreased as much as 32% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

- the timing of our submissions to regulatory authorities and regulatory decisions and developments including any potential decision by the FDA to review eteplirsen on an expedited or normal pathway, if at all;
- positive or negative results from or regulatory interpretations of testing and clinical trials by ourselves, 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 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;

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 earlier this year and appointment of an interim CEO; or

general market conditions in our industry or in the economy as a whole.

Table of Contents

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 June 30, 2015, there were 41.5 million shares of common stock outstanding and outstanding awards to purchase 7.0 million shares of common stock under various incentive stock plans. Additionally, as of June 30, 2015, there were 2.0 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, 0.2 million shares of common stock

Table of Contents

available for issuance under our 2013 Employee Stock Purchase Plan and 0.4 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.

Table of Contents

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: August 6, 2015

By: /s/ EDWARD KAYE, MD
Edward Kaye, MD
Interim Chief Executive Officer, Senior Vice President, Chief Medical Officer

(Principal Executive Officer)

Date: August 6, 2015

By: /s/ SANDESH MAHATME
Sandesh Mahatme
Senior Vice President, Chief Financial Officer

(Principal Financial and Accounting Officer)

Table of Contents

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
3.1	Amended and Restated Certificate of Incorporation of Sarepta Therapeutics, Inc.	8-K12B	001-14895	3.1	6/6/13	
3.2	Amendment to the Amended and Restated Certificate of Incorporation of Sarepta Therapeutics, Inc.	8-K	001-14895	10.1	6/30/15	
3.3	Amended and Restated Bylaws of Sarepta Therapeutics, Inc.	8-K	001-14895	3.1	9/25/14	
10.1	Credit and Security Agreement between the Company and MidCap Financial dated June 26, 2015					X
10.2	Pledge Agreement between the Company and MidCap Financial dated June 26, 2015					X
10.3	Separation and Consulting Agreement and General Release between the Company and Christopher Garabedian entered into on June 30, 2015					X
10.4	Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan	8-K	001-14895	10.1	6/16/11	
10.5	Amendment No. 1 to the Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan	8-K	001-14895	10.1	6/30/15	
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

Indicates management contract or compensatory plan, contract or arrangement.

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