Sarepta Therapeutics, Inc.
Form 10-Q
May 08, 2014
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2014

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	**40,678,439**
(Class)	**(Outstanding as of April 30, 2014)**

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SAREPTA THERAPEUTICS, INC.

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PART I FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.

(A Development Stage Company)

CONDENSED CONSOLIDATED BALANCE SHEETS

(unaudited)

(in thousands, except per share amounts)

	As of March 31, 2014	As of December 31, 2013
Assets		
Current Assets:		
Cash and cash equivalents	$ 49,101	$ 256,965
Short-term investments	176,091	
Accounts receivable	7,849	3,530
Restricted investments	7,250	7,250
Other current assets	9,079	3,061
Total Current Assets	249,370	270,806
Restricted investments	647	647
Property and equipment, net of accumulated depreciation and amortization	20,130	15,049
Patent costs, net of accumulated amortization of $1,740 and $1,622	5,217	5,042
Other assets	25	25
Total Assets	$ 275,389	$ 291,569
Liabilities and Stockholders Equity		
Current Liabilities:		
Accounts payable	$ 7,721	$ 8,080
Accrued expenses	16,340	14,601
Current portion of long-term debt	94	92
Warrant liability	9,213	9,006
Deferred revenue	3,303	3,299
Other liabilities	1,087	888
Total Current Liabilities	37,758	35,966

Long-term debt	1,551	1,576
Deferred rent and other long-term liabilities	7,118	6,835
Total Liabilities	46,427	44,377
Commitments and contingencies		
Stockholders Equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding		
Common stock, $.0001 par value, 50,000,000 shares authorized; 37,986,041 and 37,751,920 issued and outstanding at March 31, 2014 and December 31, 2013, respectively	4	4
Additional paid-in capital	800,526	790,424
Accumulated other comprehensive loss	(59)	
Deficit accumulated during the development stage	(571,509)	(543,236)
Total Stockholders Equity	228,962	247,192
Total Liabilities and Stockholders Equity	$ 275,389	$ 291,569

See accompanying notes to unaudited condensed consolidated financial statements.

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SAREPTA THERAPEUTICS, INC.

(A Development Stage Company)

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited)

(in thousands, except per share amounts)

	For the Three Months Ended March 31,		July 22, 1980 (Inception) through
	2014	2013	March 31, 2014
Revenue from license fees, grants and research contracts	$ 6,088	$ 4,474	$ 193,855
Operating expenses:			
Research and development	20,906	13,762	479,483
General and administrative	10,303	6,127	160,984
Acquired in-process research and development			29,461
Operating loss	(25,121)	(15,415)	(476,073)
Other income (loss):			
Interest income and other, net	99	237	9,948
Loss on change in warrant valuation	(3,251)	(26,906)	(92,246)
Realized gain on sale of available-for-sale securities			3,863
Write-down of available-for-sale securities			(17,001)
Total other loss	(3,152)	(26,669)	(95,436)
Net loss	$ (28,273)	$ (42,084)	$ (571,509)
Other comprehensive income (loss):			
Write-down of available-for-sale securities			17,001
Realized gain on sale of available-for-sale securities			(3,863)
Unrealized loss on available-for-sale securities	(59)		(13,197)
Total other comprehensive loss	(59)		(59)
Comprehensive loss	$ (28,332)	$ (42,084)	$ (571,568)

Net loss per share:					
Net loss per share	basic and diluted	$	(0.75)	$	(1.32)
Weighted average number of common shares outstanding for computing basic and diluted net loss per share			37,821		31,813

See accompanying notes to unaudited condensed consolidated financial statements.

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SAREPTA THERAPEUTICS, INC.

(A Development Stage Company)

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	For the Three Months Ended March 31,		For the Period July 22, 1980 (Inception) through
	2014	2013	March 31, 2014
Cash flows from operating activities:			
Net loss	$ (28,273)	$ (42,084)	$ (571,509)
Adjustments to reconcile net loss to net cash flows used in operating activities:			
Depreciation and amortization	523	451	23,770
Amortization of premium on available-for-sale securities	519		519
Loss on abandonment of patents and disposal of property and equipment	6	196	3,232
Realized gain on sale of available-for-sale securities			(3,863)
Write-down of available-for-sale securities			17,001
Impairment charge on real estate owned			1,445
Stock-based compensation	4,342	1,671	47,542
Acquired in-process research and development			29,461
Increase in warrant valuation	3,251	26,906	92,246
Net increase in accounts receivable and other assets	(10,337)	(1,178)	(16,297)
Net increase (decrease) in accounts payable, accrued expenses and other liabilities	1,704	(955)	23,585
Net cash used in operating activities	(28,265)	(14,993)	(352,868)
Cash flows from investing activities:			
Purchase of restricted investments		(7,250)	(7,897)
Purchase of property and equipment	(5,326)	(9)	(27,683)
Patent costs	(294)	(461)	(12,228)
Purchase of available-for-sale securities	(176,669)		(289,662)
Sale of available-for-sale securities			117,724
Acquisition costs			(2,389)
Net cash used in investing activities	(182,289)	(7,720)	(222,135)
Cash flows from financing activities:			
	2,713	2,993	625,258

Proceeds from exercise of options and warrants and the sale of common stock, warrants, and partnership units, net of offering costs			
Repayments of long-term debt	(23)	(22)	(542)
Other financing activities, net			(612)
Net cash provided by financing activities	2,690	2,971	624,104
Increase (decrease) in cash and cash equivalents	(207,864)	(19,742)	49,101
Cash and cash equivalents:			
Beginning of period	256,965	187,661	
End of period	$ 49,101	$ 167,919	$ 49,101
Supplemental Disclosure of Cash Flow Information			
Cash paid during the period for interest	$ 20	$ 22	$ 739
Supplemental Schedule of Noncash Investing Activities and Financing Activities:			
Available-for-sale securities received in connection with the private offering	$	$	$ 17,897
Issuance of common stock in satisfaction of warrants and other liabilities	$ 3,044	$ 1,022	$ 114,092
Tenant improvements paid by landlord	$ 593	$	$ 6,807
Property and equipment included in accounts payable and other liabilities	$ 3,533	$	$ 3,533
Issuance of common stock for building purchase	$	$	$ 750
Assumption of long-term debt for building purchase	$	$	$ 2,200
Issuance of common stock to acquire assets	$	$	$ 8,075
Assumption of liabilities to acquire assets	$	$	$ 2,124

See accompanying notes to unaudited condensed consolidated financial statements.

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SAREPTA THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. BUSINESS AND BASIS OF PRESENTATION

Business

Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries (Sarepta or the Company) is a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious diseases. Applying its proprietary platform technologies, the Company is able to target a broad range of diseases and disorders through distinct RNA-based mechanisms of action. The Company is focused on advancing the development of its Duchenne muscular dystrophy (DMD) drug candidates, including its lead product candidate, eteplirsen, for which the Company is currently conducting an ongoing open label extension study following completion of its initial Phase IIb clinical trials. The Company is also focused on developing therapeutics for the treatment of infectious diseases, including its lead infectious disease program aimed at the development of a drug candidate for the Marburg hemorrhagic fever virus for which the Company has historically received significant financial support from U.S. government research contracts.

Since its inception in 1980, the Company has incurred losses of approximately $571.5 million, substantially all of which resulted from expenditures related to research and development and general and administrative charges and loss on change in warrant valuation partially offset by revenue generated from government research contracts and other grants. The Company has not generated any material revenue from product sales to date and there can be no assurance that revenue from product sales will be achieved. Moreover, even if the Company does achieve revenue from product sales, it is likely to continue to incur operating losses in the near term.

As of March 31, 2014, the Company had $233.1 million of cash, cash equivalents and investments, consisting of $49.1 million of cash and cash equivalents, $176.1 million of short-term investments and $7.9 million of restricted investments. On April 29, 2014, the Company sold 2,650,000 shares of common stock in a public offering at a price of $38.00 per share resulting in net proceeds (after deducting underwriting discounts, commissions and other estimated offering expenses) to the Company of approximately $94.5 million. The Company believes, taking into consideration outstanding warrants and the net proceeds of $94.5 million from the common stock offering, that its balance of cash, cash equivalents and investments is sufficient to fund its current operational plan for the next twelve months. Should the Company s funding from the U.S. government cease or be delayed, the Company would likely curtail certain of its infectious disease research and development efforts unless additional funding was obtained. The Company is also likely to 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, 2013.

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 and liability classified warrants, research and development expenses and revenue recognition.

Reclassification

The Company has revised the presentation as well as the caption of certain current liabilities within the unaudited condensed consolidated balance sheets to conform to the current period presentation. Accrued liabilities of $9.6 million as of March 31, 2013, which is broken out from accounts payable , and accrued employee compensation of $5.0 million are presented as accrued expenses in the unaudited condensed consolidated balance sheets. This revision had no impact on total current liabilities or total liabilities.

2. RECENT ACCOUNTING PRONOUNCEMENTS

In July 2013, the Financial Accounting Standards Board (FASB) issued new guidance which amends the guidance related to the presentation of unrecognized tax benefits and allows for the reduction of a deferred tax asset for a net operating loss (NOL) carryforward whenever the NOL or tax credit carryforward would be available to reduce the additional taxable income or tax due if the tax position is disallowed. The new guidance is effective for annual and interim periods for fiscal years beginning after December 15, 2013, and early adoption is permitted. Since the guidance relates only to the presentation of unrecognized tax benefits, the adoption of this guidance did not have a material effect on the Company s financial position, results of operations or cash flows.

3. ACCOUNTS RECEIVABLE

The Company s accounts receivable primarily arise from government research contracts and other grants. They are generally stated at invoiced amount and do not bear interest. Because the accounts receivable are primarily from government agencies and historically no amounts have been written off, an allowance for doubtful accounts receivable is not considered necessary. The accounts receivable balance included unbilled receivables of $5.5 million and $2.4 million at March 31, 2014 and December 31, 2013, respectively. Approximately $3.8 million of the unbilled receivables as of March 31, 2014 are subject to a U.S. government audit and will not be collected until the completion of the audit.

4. FAIR VALUE MEASUREMENTS

The Company has certain financial assets and liabilities 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.

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

	Fair Value Measurement as of March 31, 2014			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Cash equivalents	$ 8,162	$ 912	$ 7,250	$
Commercial paper	24,890		24,890	
Government and government agency bonds	88,745		88,745	
Corporate bonds	62,456		62,456	
Certificates of deposit	7,897	7,897		
Total assets	$ 192,150	$ 8,809	$ 183,341	$

	Fair Value Measurement as of December 31, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Cash equivalents	$ 185,000	$ 185,000	$	$
Certificates of deposit	7,897	7,897		
Total assets	$ 192,897	$ 192,897	$	$

	Fair Value Measurement as of March 31, 2014			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants	$ 9,213	$	$	$ 9,213
Total liabilities	$ 9,213	$	$	$ 9,213

	Fair Value Measurement as of December 31, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants	$ 9,006	$	$	$ 9,006
Total liabilities	$ 9,006	$	$	$ 9,006

The cash equivalents include money market funds, commercial paper and government and government agency bonds. Money market funds are publicly traded mutual funds and, thus, the fair value of these assets is categorized as Level 1 within the fair value hierarchy.

The majority of the Company s financial assets, consisting of commercial paper, government and government agency bonds and corporate bonds, 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. Thus, the fair value of these assets is categorized as Level 2 within the fair value hierarchy.

The warrants issued in January and August 2009 are classified as liabilities. The fair value is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates for the inputs in the model. Accordingly, the fair value of these liabilities is categorized as Level 3 within the fair value hierarchy. For additional information related to the determination of fair value of warrants and a reconciliation of changes in fair value, please read *Note 7, Warrants* of the unaudited condensed consolidated financial statements.

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

5. CASH, CASH EQUIVALENTS AND SHORT-TERM INVESTMENTS

It is the Company s policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. The following tables summarize the Company s cash, cash equivalents and short-term investments for each of the periods indicated:

	As of March 31, 2014			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash, money market funds, commercial paper and government and government agency bonds	$ 49,101	$	$	$ 49,101
Commercial paper	24,892	1	(3)	24,890
Government and government agency bonds	88,755	4	(14)	88,745
Corporate bonds	62,503		(47)	62,456
Total	$ 225,251	$ 5	$ (64)	$ 225,192
As reported:				
Cash and cash equivalents	49,101			49,101
Short-term investments	176,150	5	(64)	176,091
Total	$ 225,251	$ 5	$ (64)	$ 225,192

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	As of December 31, 2013		
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 256,965	$	$	$ 256,965
Total	$ 256,965	$	$	$ 256,965
As reported:				
Cash and cash equivalents	$ 256,965	$	$	$ 256,965
Total	$ 256,965	$	$	$ 256,965

6. PROPERTY AND EQUIPMENT

Property and equipment are recorded at historical cost, net of accumulated depreciation. They are subject to impairment analysis whenever events or changes in circumstances indicate that the carrying amount of the assets may not be recoverable. The cost of normal, recurring or periodic repairs and maintenance activities related to property and equipment is expensed as incurred. The cost for planned major maintenance activities, including the related acquisition or construction of assets, is capitalized if the project will result in future economic benefits.

The following table summarizes the Company s property and equipment for each of the periods indicated:

	As of March 31, 2014	As of December 31, 2013
	(in thousands)	
Lab equipment	$ 8,332	$ 7,728
Office equipment	2,210	306
Software and computer equipment	2,955	1,126
Building	1,856	1,856
Leasehold improvements	21,359	10,058
Construction in progress	521	11,303
Property and equipment, gross	37,233	32,377
Less: accumulated depreciation	(17,103)	(17,328)
Property and equipment, net	$ 20,130	$ 15,049

At December 31, 2013, the Company recorded construction in progress related to the Cambridge headquarter lease totaling $11.3 million, of which $6.2 million was paid by the landlord as part of a tenant improvement allowance which was recorded in other long-term liabilities. These assets were placed into service, reclassified to their respective asset category and depreciation commenced during the first quarter of 2014.

Depreciation expense was $0.4 million and $0.3 million for the three months ended March 31, 2014 and 2013, respectively.

7. WARRANTS

The Company has periodically issued warrants in connection with certain common stock offerings. The warrants issued in January and August 2009 are classified as liabilities as opposed to equity due to their settlement terms which require settlement in registered shares. These warrants are non-cash liabilities and the Company is not required to expend any cash to settle these liabilities.

The outstanding warrants classified as liabilities are recorded on the unaudited condensed consolidated balance sheets and are adjusted to fair value at each financial reporting period, with changes in the fair value being recorded as Gain (loss) on change in warrant valuation in the unaudited condensed consolidated statements of operations and comprehensive loss. Fair value is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates for the inputs used in the model.

The following table reflects the weighted-average assumptions for each of the periods indicated:

	For the Three Months Ended March 31,	
	2014	2013
Risk-free interest rate (1)	0.1%	0.1%
Expected dividend yield (2)	0%	0%
Expected lives (3)	0.3-0.4 year	0.8-1.4 years
Expected volatility (4)	101.81%-121.88%	99.4%-110.7%
Shares underlying warrants classified as liabilities	633,740	3,093,676
Market value of stock at beginning of the period	$ 20.37	$ 25.80
Market value of stock at end of the period	$ 24.03	$ 36.95

(1) The risk-free interest rate is estimated using an average of U.S. Treasury bill interest rates that correlate to the prevailing interest rates at the valuation date.
(2) The expected dividend yield is zero as the Company has not paid any dividends to date and does not expect to pay dividends prior to the expiration of the warrants.
(3) The expected lives are based on the remaining contractual lives of the related warrants at the valuation date.
(4) The expected volatility is estimated using a blend of calculated volatility of the Company s common stock over a historical period and implied volatility in exchange-traded options associated with the Company s common stock.

The amounts estimated according to the Black-Scholes-Merton option-pricing model may not be indicative of the actual values realized upon the exercise of these warrants by the holders.

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The following table summarizes the reconciliation of the change in value of the Company s liability classified warrants for each of the periods indicated:

	For the Three Months Ended March 31,	
	2014	2013
	(in thousands)	
Balance at beginning of the period	$ 9,006	$ 65,193
Increase in value of warrants	3,251	26,906
Reclassification to stockholders equity upon exercise of warrants	(3,044)	(1,022)
Balance at end of the period	$ 9,213	$ 91,077

The following table summarizes the Company s warrant activity for each of the periods indicated:

	For the Three Months Ended March 31,			
	2014		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Warrants outstanding at beginning of the period	791,508	$ 10.50	3,127,618	$ 8.48
Exercised	(157,768)	10.59	(33,942)	8.16
Warrants outstanding at end of the period	633,740	$ 9.92	3,093,676	$ 8.49
Warrants exercisable at end of the period	633,740	9.92	3,093,676	8.49

The following table summarizes the Company s warrants outstanding at March 31, 2014:

Issue Date	Exercise Price	Outstanding Warrants	Expiration Date
1/30/2009	$ 6.96	129,768	7/30/2014
8/25/2009	$ 10.68	503,972	8/31/2014

8. ACCRUED EXPENSES

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

	As of March 31,	As of December 31,

	2014	2013
	(in thousands)	
Accrued contract manufacturing costs	$ 5,169	$ 1,414
Accrued facility-related costs	3,531	2,843
Accrued contract research costs	2,623	2,785
Accrued employee compensation costs	2,611	5,048
Accrued professional fees	1,606	1,235
Others	800	1,276
Total accrued expenses	$ 16,340	$ 14,601

9. GOVERNMENT CONTRACTS

The Company recognizes revenue from U.S. and European Union (E.U.) government research contracts during the period in which the related expenditures are incurred and presents revenue and related expenses gross in the unaudited condensed consolidated statements of operations and comprehensive loss. In the periods presented, substantially all of the revenue generated by the Company was derived from government research contracts.

The following table summarizes the revenue for each of our contracts with the U.S. and E.U. governments for each of the periods indicated:

	For the Three Months Ended March 31,	
	2014	2013
	(in thousands)	
July 2010 Contract (*Ebola and Marburg IV*)	$ 4,064	$ 2,614
August 2012 Contract (*Intramuscular*)		1,806
November 2012 SKIP-NMD Agreement (*DMD*)	1,365	54
July 2013 Children s National Medical Center *(DMD)*	659	
Total	$ 6,088	$ 4,474

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July 2010 Contract (Ebola and Marburg Intravenous administration)

In July 2010, the Company was awarded the Department of Defense (DoD) contract managed by its Joint Project Manager Medical Countermeasure Systems (JPM-MCS) program for the advanced development of its hemorrhagic fever virus therapeutic candidates, AVI-6002 and AVI-6003, against the Ebola and Marburg viruses, respectively. In February 2012, the Company announced that it received permission from the U.S. Food and Drug Administration (FDA) to proceed with a single oligomer from AVI-7288, one of the two components that make up AVI-6003, as the lead product candidate against Marburg virus infection. In August 2012, the Company received a stop-work order related to the Ebola virus portion of the contract and, in October 2012, the DoD terminated the Ebola portion of the contract for the convenience of the government due to government funding constraints.

The remaining Marburg portion of the contract is structured into four segments and has an aggregate remaining period of performance spanning approximately four years if DoD exercises its options for all segments. Activities under the first segment began in July 2010 and include preclinical studies and Phase I studies in healthy volunteers. In February 2014, the Company announced positive safety results from the Phase I multiple ascending dose study of AVI-7288.

After completion of the first segment, DoD has the option to proceed to the next segment. If DoD exercises its options for segments II, III and IV, the Company s contract activities would include all clinical and licensure activities necessary to obtain FDA regulatory approval for the therapeutic candidate against the Marburg virus. The funding for segments II, III and IV of the Marburg virus portion of the contract is estimated to be approximately $84.4 million.

During the three months ended March 31, 2014 and 2013, the Company recognized $4.1 million and $2.6 million as revenue under this agreement, respectively.

August 2012 Agreement (Intramuscular)

In August 2012, the Company was awarded a contract from the JPM-MCS program. The contract was for approximately $3.9 million to evaluate the feasibility of an intramuscular route of administration using AVI-7288, the Company s candidate for treatment of Marburg virus. The period of performance for this contract concluded in the third quarter of 2013. No revenue was recognized under this agreement for the three months ended March 31, 2014. The Company recognized $1.8 million as revenue under this agreement for the three months ended March 31, 2013.

European Union SKIP-NMD Agreement (DMD)

In November 2012, the Company entered into an agreement for a collaborative research project partially funded by the E.U. Health Innovation. The agreement provides for approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic and is expected to last approximately three years. During the three months ended March 31, 2014 and 2013, the Company recognized $1.4 million and less than $0.1 million as revenue under this agreement, respectively. Revenue under this agreement has been fully recognized as March 31, 2014.

July 2013 Children s National Medical Center (CNMC) Agreement (DMD)

In July 2013, the Company entered into an agreement totaling $1.3 million to provide a drug product to CNMC to conduct research related to the Company s DMD program. During the three months ended March 31, 2014, the Company recognized $ 0.7 million as revenue under the agreement. Revenue under this agreement has been fully recognized as March 31, 2014.

10. STOCK-BASED COMPENSATION

The Company s equity incentive plans allow for the granting of a variety of stock awards. To date, the Company has granted stock options, restricted stock awards, restricted stock units and stock appreciation rights. The fair value of stock awards, with consideration given to estimated forfeitures, is recognized as compensation expense on a straight-line basis over the vesting period of the grants.

Stock Options

The Company has granted stock options with both service- and performance-based criteria. In general, stock options granted vest over four years and have a ten-year term. As of March 31, 2014, the achievement of the performance criteria is not probable and, accordingly, the Company has not recognized any expense related to the options with performance-based criteria.

The following table summarizes the Company s stock option activity for each of the periods indicated:

	For the Three Months Ended March 31,			
	2014		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	4,190,367	$ 23.46	2,522,522	$ 11.76
Granted	1,185,265	26.83	242,320	28.76
Exercised	(48,579)	10.39	(64,390)	8.97
Canceled or expired	(53,715)	30.26	(5,774)	14.83
Grants outstanding at end of the period	5,273,338	$ 24.27	2,694,678	$ 13.35
Grants exercisable at end of the period	1,180,245	$ 12.85	656,805	$ 12.36

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The fair values of stock options granted during the periods presented were measured on the date of grant using the Black-Scholes-Merton option-pricing model, with the following assumptions:

	For the Three Months Ended March 31,	
	2014	2013
Risk-free interest rate (1)	1.7%	0.7%
Expected dividend yield (2)	0%	0%
Expected lives (3)	4.9 years	4.9 years
Expected volatility (4)	95.4%	80.0%

(1) The risk-free interest rate is estimated using an average of Treasury bill interest rates over a historical period commensurate with the expected term of the option that correlates to the prevailing interest rates at the time of grant.
(2) The expected dividend yield is zero as the Company has not paid any dividends to date and does not expect to pay dividends in the future.
(3) The expected lives are estimated using expected and historical exercise behavior.
(4) The expected volatility is estimated using a blend of calculated volatility of the Company s common stock over a historical period and implied volatility in exchange-traded options of the Company s common stock.

The amounts estimated according to the Black-Scholes-Merton option-pricing model may not be indicative of the actual values realized upon the exercise of these options by the holders.

Restricted Stock Awards (RSA)

The Company grants RSAs to members of its board of directors. The weighted-average grant date fair value of RSAs is based on the market price of the Company s common stock on the date of grant. The fair value is amortized to stock-based compensation expense on a straight-line basis over the vesting period of the grants. The following table summarizes the Company s RSA activity for each of the periods indicated:

	For the Three Months Ended March 31,			
	2014		2013	
	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value
Grants outstanding at beginning of the period	6,000	$ 34.92	4,998	$ 10.08
Granted	6,000	29.03		
Grants outstanding at end of the period	12,000	$ 31.98	4,998	$ 10.08

Restricted Stock Units (RSU)

The Company granted RSUs to employees in 2012. The weighted-average grant date fair value of RSUs is based on the market price of the Company s common stock on the date of grant. The fair value is amortized to stock-based compensation expense on a straight-line basis over the vesting period of the grants. The following table summarizes the Company s RSU activity for each of the periods indicated:

	For the Three Months Ended March 31,			
	2014		2013	
	Shares	Weighted Average Grant Date Fair Value	Shares	Weighted Average Grant Date Fair Value
Grants outstanding at beginning of the period	6,507	$ 5.40	38,260	$ 6.32
Canceled or expired			(262)	5.40
Grants outstanding at end of the period	6,507	$ 5.40	37,998	$ 6.33

Stock Appreciation Rights (SAR)

The Company issues SARs to employees on the same terms as the stock options granted to employees. The grant date fair value of the SARs is determined using the same valuation assumptions as for the stock options described above. Stock-based compensation expense is recognized on a straight-line basis over the vesting period of the SARs. The following table summarizes the Company s SAR activity for each of the periods indicated:

	For the Three Months Ended March 31,			
	2014		2013	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	170,000	$ 18.18	170,000	$ 18.18
Grants outstanding at end of the period	170,000	$ 18.18	170,000	$ 18.18
Grants exercisable at end of the period	61,041	$ 17.60		$

Employee Stock Purchase Plan (ESPP)

Under the Company s ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company s common stock on the first business day and the last business day of the relevant plan period. The 24-month award period will end on February 29, 2016. For the purchasing period ended February 28, 2014, 21,774 shares of the Company s common stock were purchased for total proceeds of $0.5 million.

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The fair values of stock purchase rights were estimated using the Black-Scholes-Merton option-pricing model. The following table summarizes the assumptions used in the calculation during the period indicated:

	For the Three Months Ended March 31, 2014
Fair value of stock purchase rights	$12.22 - 25.35
Weighted-average stock price volatility	101% - 109%
Expected lives	0.5 - 2 years
Risk-free rate	0.1% - 0.3%

Stock-based Compensation Expense

For the three months ended March 31, 2014 and 2013, total stock-based compensation expense was $4.3 million and $1.7 million, respectively. The following table summarizes share-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended March 31,	
	2014	2013
Research and development	$ 1,873	$ 530
General and administrative	2,469	1,141
Total	$ 4,342	$ 1,671

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

	For the Three Months Ended March 31,	
	2014	2013
Stock options	$ 3,911	$ 1,401
Restricted stock awards	64	12
Restricted stock units	(4)	117
Stock appreciation rights	147	141
Employee stock purchase plan	224	
Total	$ 4,342	$ 1,671

11. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. Given that the Company is in a loss position 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 are, therefore, excluded from the diluted net loss per share calculation.

	For the Three Months Ended March 31,	
	2014	2013
	(in thousands, except per share amounts)	
Net loss	$ (28,273)	$ (42,084)
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 earnings per share	37,821	31,813
Dilutive effect of outstanding warrants 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 earnings per share	37,821	31,813
Net loss per share basic and diluted	$ (0.75)	$ (1.32)

* Warrants, stock options, restricted stock awards, restricted stock units and stock appreciation rights to purchase approximately 6,096,000 and 5,997,000 shares of common stock were excluded from the net loss per share calculation for the three months ended March 31, 2014 and 2013, respectively, as their effect would have been anti-dilutive.

12. INCOME TAXES

As of December 31, 2013, the Company had gross deferred tax assets of $133.6 million primarily from U.S. federal and state NOL carryforwards, U.S. federal and state research and development credit carryforwards, stock-based compensation expense and intangibles. Due to uncertainties surrounding the Company s ability to generate future taxable income to realize these assets, a full valuation allowance has been established to offset its net deferred tax asset. Additionally, the Internal Revenue Code rules could limit the future use of its NOL and research and development credit carryforwards to offset future taxable income based on ownership changes and the value of the Company s common stock.

13. RESTRUCTURING

In November 2012, the Company notified 21 Bothell, Washington based employees that they would be terminated as part of the corporate headquarters relocation to Cambridge, Massachusetts. The employees were given various incentives to remain through a transition period which was completed in 2013. During the first quarter of 2014, the transition period was extended to the end of the second quarter of 2014 for one employee. For the three months ended March 31, 2014, the Company recorded a restructuring charge of less than $0.1 million to research and development expenses. For the three months ended March 31, 2013, the Company recorded a restructuring charge of $0.3 million to research and development expenses and $0.2 million to general and administrative expenses.

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Changes in the liability and the balance related to the restructuring plan are as follows:

	For the Three Months Ended March 31,	
	2014	2013
	(in thousands)	
Balance at beginning of the period	$ 44	$ 185
Restructuring charges	9	462
Payments	(8)	(201)
Balance at end of the period	$ 45	$ 446

14. COMMITMENTS AND CONTINGENCIES

Lease Obligations

In June 2013, the Company entered into a lease agreement for its headquarters located in Cambridge, Massachusetts. The agreement calls for a security deposit in the form of a letter of credit totaling $0.6 million. The Company purchased a certificate of deposit (CD) to meet the requirement and it was recorded as a long-term restricted investment in the unaudited condensed consolidated balance sheets as of March 31, 2014. The initial term of the lease agreement is for seven years with an average annual base rent of approximately $2.4 million.

In November 2013, the Company entered into the first amendment of its lease agreement for its headquarters located in Cambridge, Massachusetts. The amendment modified the original lease to add an additional 15,077 square feet to its original space, increasing its total rental space for its headquarters to 61,453 square feet. The amendment calls for additional annual base rent of approximately $0.5 million, subject to a 2.5% annual increase. In January 2014, the Company entered into a sublease for the 15,077 square feet of office space with an unrelated third party for an 18-month term with a total base rent of approximately $0.7 million.

The Company also leases laboratory and office space in Corvallis, Oregon. The annual base rent at the Corvallis, Oregon facility is approximately $0.9 million, excluding other occupancy costs, and is subject to an annual increase of 3%.

For the three months ended March 31, 2014 and 2013, rent expense and occupancy costs under all leases totaled $1.1 million and $0.6 million, respectively. The aggregate non-cancelable future minimum payments under leases were as follows:

	As of March 31, 2014 (in thousands)
2014 (9 months)	$ 2,790
2015	3,807
2016	3,895
2017	3,986
2018	4,079

Thereafter		8,889
Total minimum lease payments	$	27,446

Royalty Obligations

The Company has license agreements for which it is obligated to pay minimum royalties if the Company does not terminate the relevant agreement. The notice period to terminate these agreements is six months or less. Royalty payments under these agreements were less than $0.1 million for the three months ended March 31, 2014 and 2013, respectively.

The Company is also obligated to pay royalties upon the net sales of DMD products. The royalty rates are in the low single-digit percentages for both inside and outside the United States. Under the agreement with Charley s Fund, Inc. signed in October 2007, the Company is obligated to pay a mid single-digit percentage royalty on the net sales of any product developed pursuant to the agreement with Charley s Fund up to a maximum of $3.4 million. In May 2003, the Company entered into a collaboration and license agreement with Isis-Ercole. The range of percentage of royalty payments under this agreement, should such payments ever be made, is from a fraction of a percent to mid-single-digit percentages.

Milestone Obligations

The Company has license agreements for which it is obligated to pay development milestones as a product candidate proceeds from the filing of an Investigational New Drug application through approval for commercial sale. There were no milestone payments under these agreements for the three months ended March 31, 2014 and 2013, respectively.

Under the collaboration and license agreement with Isis-Ercole, the Company may be obligated to make up to $23.4 million milestone payments. As of March 31, 2014, the Company has not made any payments under this agreement and is not under any current obligation to make any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied. Subject to the satisfaction of certain milestones triggering the obligation to make any such payments, Isis may be obligated to make milestone payments to the Company of up to $21.1 million in the aggregate for each product developed under a licensed patent under this agreement. As of March 31, 2014, Isis has not made and is not under any current obligation to make any such milestone payments, as the conditions triggering any such milestone payment obligations have not been satisfied.

In April 2013, the Company and the University of Western Australia (UWA) entered into an agreement under which an existing exclusive license agreement between the Company and UWA was amended and restated. Under the terms of this agreement, UWA granted the Company an exclusive license to certain UWA intellectual property rights in exchange for up to $7.1 million in upfront and development milestone payments.

In March 2014, the Company entered into a patent assignment agreement with a group of scientists (collectively, Assignors). Under the terms of the agreement, the Assignors will transfer to the Company all rights, title and interest in certain patent rights as well as technical information related to the patents. The Company may be obligated to make up to $2.7 million in development and commercial milestone payments. As of March 31, 2014, the Company made an up-front payment of $0.3 million under this agreement, which was recorded as research and development expense.

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, in January 2014, a former consultant of the Company

filed a complaint alleging breach of contract, among other claims, and seeking approximately $4 million in damages, plus certain additional fees and costs, from

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the Company. In addition, 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 (Corban v. Sarepta et al) and January 29, 2014 (Baradanian v. Sarepta et al). The plaintiffs are alleged purchasers of Company common stock who seek to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 24, 2013 and November 12, 2013. The complaints allege that the defendants violated the federal securities laws in connection with disclosures related to eteplirsen, the Company s lead therapeutic candidate for DMD, and seek damages in an unspecified amount. Given the relatively early stages of the proceedings in the above-mentioned purported claims, at this time, no assessment can be made as to the likely outcome of these claims or whether the outcomes would have a material impact on the Company.

Purchase Commitments

In the Company s continuing operations, it has entered into long-term contractual arrangements from time to time for the provision of goods and services. The following table presents non-cancelable contractual obligations arising from these arrangements:

	As of March 31, 2014 (in thousands)
2014 (9 months)	$ 38,528
2015	44,901
2016	42,779
2017	21,390
2018	14,260
Thereafter	3,565
Total purchase commitments	$ 165,423

In February 2013, the Company issued two letters of credit totaling $7.3 million to a contract manufacturing vendor in connection with certain manufacturing agreements. The obligations secured by the letters of credit are fulfilled upon payment for certain minimum volume commitments and construction milestones. To meet the requirement of the letters of credit, the Company purchased $7.3 million in CDs with May 2014 maturity dates. If the minimum volume commitments and construction milestones have not occurred at that time, the letters of credit will be extended. The $7.3 million was recorded as restricted investments in the unaudited condensed consolidated balance sheets as of March 31, 2014.

15. SUBSEQUENT EVENT

On April 29, 2014, the Company sold 2,650,000 shares of common stock at an offering price of $38.00 per share. In addition, the Company has granted the underwriters a 30-day option to purchase an additional 397,500 shares of common stock at a price of $38.00 per share. The Company received aggregate net proceeds of approximately $94.5 million, after deducting the underwriting discounts and offering-related transaction costs.

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, 2013 under the caption Part II-Item 7 Management s Discussion and Analysis of Financial Condition and Results of Operations . This discussion contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements are identified by words such as believe, anticipate, expect, intend, plan, will, may, seek and other similar expressions. 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 development and clinical benefits of our product candidates;

the results of our research and development efforts and the efficacy of our phosphorodiamidate-linked morpholino oligomer (PMO) chemistries and other RNA-based technology-based chemistries and other RNA-based technology;

our expectations regarding our ability to become a leading developer and marketer of RNA-based therapeutics;

the efficacy, potency and utility of our product candidates in the treatment of rare and infectious diseases, and their potential to treat a broad number of human diseases;

our expectations regarding the results of preclinical and clinical testing of our product candidates;

our expectations regarding the timing for completion and expected results of our product development plans, including running various clinical studies in support of and the filing of a new drug application (NDA) for eteplirsen with the approval of the U.S. Food and Drug Administration (FDA);

our expectations regarding the timing, completion and receipt of results from our ongoing development programs for our pipeline of product candidates;

the timing of and requirements the Company must comply with to receive any required approvals from the FDA or other regulatory approvals for our product candidates outside of the United States;

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

our expectations regarding the markets for our product candidates;

acceptance of our product candidates, if introduced, in the marketplace;

the possible impact of competitive products, product development, manufacturing, commercialization and technological difficulties;

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our expectations regarding partnering opportunities and other strategic transactions;

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

our plans to file additional patent applications to enhance and protect our existing intellectual property portfolio;

our ability to invalidate some or all of the claims covered by patents issued to competitors and the impact of those claims on our ability to commercialize our product candidates;

our ability to anticipate the actions and positions that the United States Patent and Trademark Office may take with respect to our patent claims or those of third parties;

our estimates regarding how long our currently available cash, cash equivalents and investments will be sufficient to finance our operations and statements about our future capital needs;

our ability to increase the scale of our manufacturing to provide our product candidates to patients in larger scale clinical trials or in potential commercial quantities and meet regulatory and company quality control requirements;

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

our expectations about funding from government and other sources; and

other factors set forth below under the heading Risk Factors .

These forward-looking statements are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements. Factors that might cause such a difference include, but are not limited to, those discussed in this Quarterly Report in Part II, Item 1A Risk Factors, and elsewhere in this Quarterly Report. These statements, like all statements in this Quarterly Report, speak only as of their date, and we undertake no obligation to update or revise these statements in light of future developments. In this report, we, our, us, Sarepta, and Company refers to Sarepta Therapeutics, Inc. and its subsidiaries.

Overview

We are a biopharmaceutical company focused on the discovery and development of unique RNA-based therapeutics for the treatment of rare and infectious 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-based mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially

disease-modifying Duchenne muscular dystrophy (DMD) drug candidates, including our lead product candidate, eteplirsen. We are also focused on developing therapeutics for the treatment of infectious diseases, including our lead infectious disease program aimed at the development of a drug candidate for the Marburg hemorrhagic fever virus. By building our infectious disease programs primarily funded and supported by the U.S. Department of Defense (DoD) and leveraging our highly-differentiated and proprietary technology platforms, we are seeking to further develop our research and development competencies and identify additional product candidates.

Our highly-differentiated RNA-based 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. Eteplirsen is our lead therapeutic candidate for DMD. If we are successful in our development efforts, eteplirsen will address a severe but unmet medical need. In 2012, we completed a U.S.-based Phase IIb clinical trial for eteplirsen that was initiated in August 2011. Following completion of this study in early 2012, we initiated an open label extension study with the same participants from the original Phase IIb placebo-controlled trial. On April 21, 2014, we announced our plans to file an NDA for eteplirsen for the treatment of DMD by the end of 2014. Additionally, we are working with the FDA to start several additional clinical studies with eteplirsen in exon 51 amenable genotypes and a placebo-controlled study with one or more of our follow-on DMD exon-skipping drug candidates by the end of 2014.

We are also leveraging the capabilities of our RNA-based technology platforms to develop therapeutics for the treatment of infectious diseases. The DoD has provided significant financial support in the past for the development of therapeutics against Ebola, Marburg, Dengue and influenza viruses. We have attracted DoD s support based in part on our ability to rapidly respond to pathogenic threats by quickly identifying, manufacturing and evaluating novel therapeutic candidates.

The basis for our novel RNA-based therapeutics is our PMO chemistries. Unlike other RNA-based therapeutics, which are often used to down-regulate gene expression, our technologies can be used to selectively up-regulate or down-regulate the production of a target protein, or direct the expression of novel proteins involved in human diseases and disorders. Further, we believe the charge-neutral nature of our PMO-based molecules may have the potential to reduce off-target effects, such as immune stimulatory effects often seen in alternative RNA-based technologies. We believe that our highly-differentiated, novel proprietary and innovative RNA-based technology platforms, based on charge-neutral morpholino oligomers, may represent a significant improvement over traditional RNA-based technologies.

Since our inception in 1980, we have incurred losses of $571.5 million, substantially all of which resulted from expenditures related to research and development, general and administrative charges and losses on changes in warrant valuation partially offset by revenue generated from government research contracts and other grants. As of March 31, 2014, we have completed all of our contracts with the DoD except for the July 2010 contract for the development of therapeutics against the Marburg virus. In November 2012, we also entered into a consortium agreement with various parties that received a grant from the European Union (E.U.) Health Innovation. We have not generated any material revenue from product sales to date, and there can be no assurance that revenue from product sales will be achieved. Moreover, even if we do achieve revenue from product sales, we are likely to continue to incur operating losses in the near term.

As of March 31, 2014, we had $233.1 million of cash, cash equivalents and investments, comprised of $49.1 million of cash and cash equivalents, $176.1 million of short-term investments and $7.9 million of restricted investments. Together with the net proceeds of $94.5 million (after deducting underwriting discounts and offering expenses) from the sale in the underwritten public offering of 2,650,000 shares of our common stock at the price of $38.00 per share on April 29, 2014, we believe our cash, cash equivalents and investments, taking into consideration of our outstanding warrants, are sufficient to fund our current operational plan for the next twelve months. Should our funding from the

U.S. government cease or be delayed, we would likely curtail certain infectious disease research and development efforts unless additional funding was obtained. We are also likely to pursue additional cash resources through public or private financings, seek additional government contracts and establish collaborations or license our technology to other companies.

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

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Government Contracts

We recognize revenue from government contracts during the period in which the related expenditures are incurred and present the revenue and related expenses gross in the unaudited condensed consolidated financial statements. In the periods presented, substantially all of the revenue generated was derived from government contracts and other grants.

The following table summarizes the revenue from each of our contracts with the U.S. and E.U. governments for the each of the periods indicated:

	For the Three Months Ended March 31,	
	2014	2013
	(in thousands)	
July 2010 Contract *(Ebola and Marburg IV)*	$ 4,064	$ 2,614
August 2012 Contract (*Intramuscular*)		1,806
November 2012 SKIP-NMD Agreement *(DMD)*	1,365	54
July 2013 Children s National Medical Center *(DMD)*	659	
Total	$ 6,088	$ 4,474

July 2010 Contract (Ebola and Marburg Intravenous administration)

In July 2010, we were awarded the DoD contract managed by its Joint Project Manager Medical Countermeasure Systems (JPM-MCS) program for the advanced development of our hemorrhagic fever virus therapeutic candidates, AVI-6002 and AVI-6003, against the Ebola and Marburg viruses, respectively. In February 2012, we announced that we received permission from the FDA to proceed with a single oligomer from AVI-7288, one of the components of AVI-6003, as the lead product candidate against Marburg virus infection. In August 2012, we received a stop-work order related to the Ebola virus portion of the contract and, in October 2012, the DoD terminated the Ebola portion of the contract for the convenience of the government due to government funding constraints.

The remaining Marburg portion of the contract is structured into four segments and has an aggregate remaining period of performance spanning approximately four years if DoD exercises its options for all segments. Activities under the first segment began in July 2010 and include preclinical studies and Phase I studies in healthy volunteers. In February 2014, we announced positive safety results from the Phase I multiple ascending dose study of AVI-7288.

After completion of the first segment, DoD has the option to proceed to the next segment. If DoD exercises its options for segments II, III and IV, our contract activities would include all clinical and licensure activities necessary to obtain FDA regulatory approval for the therapeutic candidate against the Marburg virus. The funding for segments II, III and IV of the Marburg virus portion of the contract is estimated to be approximately $84.4 million.

During the three months ended March 31, 2014 and 2013, we recognized $4.1 million and $2.6 million as revenue under this agreement, respectively.

August 2012 Agreement (Intramuscular)

In August 2012, we were awarded a contract from the JPM-MCS program. The contract was for approximately $3.9 million to evaluate the feasibility of an intramuscular route of administration using AVI-7288, our candidate for treatment of Marburg virus. The period of performance for this contract concluded in the third quarter of 2013. No revenue was recognized under this agreement for the three months ended March 31, 2014. We recognized $1.8 million as revenue under this agreement for the three months ended March 31, 2013.

European Union SKIP-NMD (SKIP-NMD) Agreement (DMD)

In November 2012, we entered into an agreement for a collaborative research project partially funded by the E.U. Health Innovation. The agreement provides for approximately $2.5 million for research in certain development and study related activities for a DMD therapeutic and is expected to last approximately three years. During the three months ended March 31, 2014 and 2013, we recognized $1.4 million and less than $0.1 million as revenue under this agreement, respectively. Revenue under this agreement has been fully recognized as March 31, 2014.

July 2013 Children s National Medical Center (CNMC) Agreement (DMD)

In July 2013, we entered into an agreement totaling $1.3 million to provide a drug product to CNMC to conduct research related to our DMD program. During the three months ended March 31, 2014, we recognized $0.7 million as revenue under the agreement. Revenue under this agreement has been fully recognized as March 31, 2014.

Key Financial Metrics

Revenue

Government Contract Revenue. Substantially all of our revenue is generated from government research contracts and other grants. We recognize revenue from government research contracts and other grants during the period in which the related expenses are incurred and present such revenue and related expenses gross in the unaudited condensed consolidated financial statements.

License Arrangements. Our license arrangements may consist of non-refundable upfront license fees, data transfer fees, research reimbursement payments, exclusive licensed rights to patented or patent pending compounds, technology access fees, various performance or sales milestones and future product royalty payments. Some of these arrangements are multiple element arrangements.

We defer recognition of non-refundable upfront 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, then such up-front fees are deferred and recognized over the period of continuing involvement. As of March 31, 2014, we had deferred revenue of $3.3 million, which represents up-front fees which we will recognize as revenue as we satisfy the outstanding performance obligations.

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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 the clinical programs. Indirect costs of our clinical program include salaries, stock-based compensation and an allocation of our facility costs.

The amount and timing of future research and development expenses will depend in part on our ability to obtain U.S. government awards to fund the advanced development of our infectious disease therapeutic candidates. Without such funding, we would likely significantly reduce our spending in these areas. Future research and development expenses may also increase as our internal projects, such as eteplirsen for DMD, enter later stage clinical development. Our research and development programs are currently in Phase IIb clinical trials or earlier and 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 ineffective during clinical trials, may take longer to complete clinical trials 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 timeframe for development of any product candidate, associated development costs and the probability of regulatory and commercial success vary widely.

General and Administrative. General and administrative expenses consist principally of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, human resource 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 (Expense) and Other, Net. Interest income (expense) and other, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense and rental income and loss. Our cash equivalents and investments consist of commercial paper, government and government agency debt securities, money market investments and certificates of deposit. Interest expense includes interest paid on our mortgage loan related to the Corvallis property, the substantial portion of which we leased to a third party in November 2011. Rental income and loss is from subleasing excess space in some of our facilities.

Gain (Loss) on Change in Warrant Valuation. Warrants issued in connection with our January and August 2009 financings are classified as liabilities as opposed to equity due to their settlement terms. These warrants are non-cash liabilities and we are 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 are marked to market each financial reporting period, with changes in the fair value recorded as Gain (loss) on change in warrant liability in our unaudited condensed consolidated statements of operations and comprehensive loss. The fair value of the warrants is determined using the Black-Scholes-Merton option-pricing model, which requires the use of significant judgment and estimates related to the inputs used in the model and can result in significant swings in the fair value primarily due to changes in our stock price. For more information, please read *Note 7, Warrants* of the unaudited

condensed consolidated financial statements contained in Part I, Item 1 of this report.

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
- accounting for and valuation of liability classified warrants.

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, 2013 filed with the Securities and Exchange Commission (SEC) on March 3, 2014.

Results of Operations for the Three Months Ended March 31, 2014 and 2013

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

	For the Three Months Ended March 31,		%
	2014	2013	Change
	(in thousands, except per share amounts)		
Revenue	$ 6,088	$ 4,474	36 %
Operating expenses:			
Research and development	20,906	13,762	52%
General and administrative	10,303	6,127	68%

Operating loss		(25,121)	(15,415)	63%
Other income (loss):				
Interest income and other, net		99	237	(58)%
Loss on change in warrant liability		(3,251)	(26,906)	(88)%
Net loss		$ (28,273)	$ (42,084)	(33)%
Net loss per share	basic and diluted	$ (0.75)	$ (1.32)	(43)%

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Revenue

Revenue for the three months ended March 31, 2014 increased by $1.6 million, or 36%, compared to the three months ended March 31, 2013. The increase was primarily due to $1.5 million of pre-clinical and clinical activities of the Marburg portion of the July 2010 agreement, $1.3 million of clinical activities of the SKIP-NMD agreement and $0.7 million from the CNMC agreement which started in July 2013. The increase was partially offset by a decrease of $1.8 million in the August 2012 agreement which was completed in the third quarter of 2013.

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 are not tracked by project, as the costs may benefit multiple projects.

Research and development expenses for the three months ended March 31, 2014 increased by $7.1 million, or 52%, compared to the three months ended March 31, 2013. The increase was primarily due to an increase of $2.5 million in headcount-related expenses, $2.5 million in our external spend in DMD programs and $0.3 million external consulting fees to prepare for the NDA for eteplirsen and grow our DMD franchise. Additionally, there was an increase of $1.2 million in rent and occupancy expenses due to construction of our new headquarters in Cambridge, Massachusetts and $0.3 million of expense recognized in connection with an up-front payment on a patent assignment agreement. The increase was partially offset by a decrease of $0.7 million in our infectious disease programs primarily due to the completion of the August 2012 agreement in the third quarter of 2013.

Prior to January 1, 2011, we did not track research and development expenditures on a project level, as such, the inception-to-date expenses represent the period from January 1, 2011 to March 31, 2014. The following table summarizes our research and development expenses for the periods indicated:

	For the Three Months Ended March 31,		%	January 1, 2011 to March 31,
	2014	2013	Change	2014
	(in thousands)			
DMD	$ 8,861	$ 6,339	40%	$ 74,973
Infectious diseases	2,011	2,734	(26)%	$ 58,684
Internal research and development costs	10,034	4,689	114%	
Total research and development expenses	$ 20,906	$ 13,762	52%	

General and Administrative Expenses

General and administrative expenses for the three months ended March 31, 2014 increased by $4.2 million, or 68%, compared to the three months ended March 31, 2013. The increase in general and administrative expenses is primarily due to $2.6 million headcount-related expenses and $0.3 million in external consulting fees to support the growth of our DMD franchise and $0.6 million in legal fees.

Interest Income and Other, Net

Interest income and other, net, for the three months ended March 31, 2014 decreased by $0.1 million, or 58%, primarily due to a realized gain during the three months ended March 31, 2013.

Loss on Change in Warrant Valuation

The loss on change in fair value of our warrant liability for the three months ended March 31, 2014 decreased by $23.7 million, or 88%, compared to the three months ended March 31, 2013. The decrease was primarily due to the fluctuation in our stock price as well as decrease in the number of our outstanding warrants. For more information, please read *Note 7, Warrants* of the unaudited condensed consolidated financial statements contained in Part I, item 1 of this report, Form 10-Q for the quarterly period ended March 31, 2014.

Net Loss

Net loss for the three months ended March 31, 2014 was $28.3 million, compared to a net loss of $42.1 million for the three months ended March 31, 2013. The decrease in net loss was primarily due to a decrease in loss on change in warrant valuation and slightly higher revenue offset by growth in both research and development and general and administrative expenses.

Liquidity and Capital Resources

At March 31, 2014, cash, cash equivalents and short-term investments were $225.2 million, compared to $257.0 million at December 31, 2013. The decrease during the three months ended March 31, 2014 is due primarily to cash used to fund operations of $28.3 million and the purchase of property and equipment of $5.3 million offset by proceeds from the exercise of warrants and stock options and Employee Stock Purchase Plan (ESPP) purchases of $2.7 million. Based on the factors described below, we believe, taking into consideration outstanding warrants and the net proceeds of $94.5 million from the common stock offering, that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for the next twelve months.

Our principal sources of liquidity are revenue from our government contracts and other grants and equity transactions. Our principal uses of cash are research and development expenses, general and administrative expenses and other working capital requirements.

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Our primary source of revenue is from development of product candidates pursuant to our government contracts and other grants. U.S. government funding is subject to the government s appropriations process and it has the right to terminate such contracts for convenience as was done regarding the Ebola portion of the July 2010 Ebola and Marburg contract in 2012. If the U.S. government funding is not received or is delayed, we would likely curtail certain of our infectious disease research and development efforts unless additional funding was obtained. Currently, we do not generate any revenue from the commercial sale of our pharmaceutical product candidates.

Our future expenditures and capital requirements depend on numerous factors, most of which are difficult to project beyond the short term. These requirements include the progress of our research and development programs and our pre-clinical and clinical trials, our ability to meet the requirements of our U.S. and E.U. government research projects, the time and costs involved in obtaining regulatory approvals, the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, competing technological and market developments, our ability to establish collaborative arrangements and the terms of any such arrangements and the costs associated with manufacturing and commercialization of our products.

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 assure you that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Three Months Ended March 31,	
	2014	**2013**
	(in thousands)	
Cash provided by (used in):		
Operating activities	$ (28,265)	$ (14,993)
Investing activities	(182,289)	(7,720)
Financing activities	2,690	2,971
Decrease in cash and cash equivalents	$ (207,864)	$ (19,742)

Operating Activities. The increase in the amount of cash used in operating activities of $13.3 million for the three months ended March 31, 2014 compared to the three months ended March 31, 2013 was primarily due to an increase in operating loss of $9.7 million driven by the growth in research and development costs and general and administrative costs and an unfavorable change in operating assets and liabilities of $6.5 million offset by an increase of $2.7 million in stock-based compensation costs, which is a non-cash adjustment to net loss.

Investing Activities. The increase in the amount of cash used in investing activities of $174.6 million for the three months ended March 31, 2014 compared to the three months ended March 31, 2013 was primarily due to the purchase of short-term investments of $176.7 million and property and equipment of $5.3 million partially offset by the purchase of restricted investment of $7.3 million during the first quarter of 2013.

Financing Activities. The decrease in the amount cash from financing activities of $0.3 million for the three months ended March 31, 2014 compared to the three months ended March 31, 2013 was primarily due to net proceeds of $2.1 million from approximately 87,000 shares of common stock under our ATM sales agreement in January 2013 offset by an increase of $1.4 million in proceeds from exercise of warrants and proceeds of $0.5 million from our ESPP program.

Contractual Obligations and Contingencies

In our continuing operations, we have entered into long-term contractual arrangements for our facilities, the provision of goods and services, and acquisition of technology access rights, among others. The following table presents non-cancelable contractual obligations arising from these arrangements as of March 31, 2014:

	Payments Due by Period				
	Total	Less Than 1 Year	1-3 Years	3-5 Years	More Than 5 Years
	(in thousands)				
Long-term debt	$ 2,208	$ 171	$ 343	$ 343	$ 1,351
Operating leases	27,446	3,742	7,747	8,112	7,845
Purchase obligations (1)	165,423	51,346	85,557	28,520	
Total	$ 195,077	$ 55,259	$ 93,647	$ 36,975	$ 9,196

(1) Purchase obligations include agreements to purchase goods or services that are enforceable and legally binding to us and that specify all significant terms. Purchase obligations relate primarily to our DMD development program.

Off-Balance Sheet Arrangements

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

Recent Accounting Pronouncements

For additional information, please read *Note 2, Recent Accounting Pronouncements* of the unaudited condensed consolidated financial statements contained in Part I, Item 1 of this report, Form 10-Q for the quarterly period ended March 31, 2014.

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Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our current investment policy is to maintain a diversified investment portfolio consisting money market investments, government and government agency bonds and high-grade corporate bonds with maturities of twenty-four months or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of March 31, 2014, we had $233.1 million of cash, cash equivalents and investments, comprised of $49.1 million of cash and cash equivalents, $176.1 million of short-term investments and $7.9 million of restricted investments. Our cash equivalents and short-term investments consist of commercial paper, government and government agency debt securities, corporate bonds and money market investments. The fair value of cash equivalents and short-term investments is subject to change as a result of potential changes in market interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. As of March 31, 2014, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of less than $0.1million to our interest rate sensitive instruments.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this report, Form 10-Q for the quarterly period ended March 31, 2014, under the supervision and with the participation of our management, including our chief executive officer and our chief financial officer, of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934 (the Exchange Act). The purpose of this evaluation was to determine whether as of the evaluation date our disclosure controls and procedures were effective to provide reasonable assurance that the information we are required to disclose in our filings with the SEC under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC s rules and forms and (ii) is accumulated and communicated to our management, including our chief executive officer and our chief financial officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of March 31, 2014, our disclosure controls and procedures were effective.

Changes in Internal Control Over Financial Reporting

As of December 31, 2013, a material weakness in our internal controls over financial reporting was identified as follows: The Company did not design and implement controls to adequately review and consider the recognition and measurement of new significant research and development contracts. During the first quarter of 2014, we designed and implemented controls to adequately review and consider the recognition and measurement of new significant research and development contracts. Such contracts are timely reviewed by our accounting personnel with the requisite accounting knowledge, skills, and experience deemed necessary to perform such a review.

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, in January 2014, a former consultant of the Company filed a complaint alleging breach of contract, among other claims, and seeking approximately $4 million in damages, plus certain additional fees and costs, from the Company. In addition, 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 (Corban v. Sarepta et al) and January 29, 2014 (Baradanian v. Sarepta et al). The plaintiffs are alleged purchasers of Company common stock who seek to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 24, 2013 and November 12, 2013. The complaints allege that the defendants violated the federal securities laws in connection with disclosures related to eteplirsen, the Company s lead therapeutic candidate for DMD, and seek damages of an unspecified amount. Given the relatively early stages of the proceedings in the above mentioned purported claims, at this time, no assessment can be made as to the likely outcome of these claims or whether the outcomes would have a material 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

Our product candidates are at an early stage of development, and it is possible that none of our product candidates will ever become commercial products.

Our product candidates are in relatively early stages of development. These product candidates will require significant further development, financial resources and personnel to obtain regulatory approval and develop into commercially viable products, if at all. Currently, eteplirsen in DMD, AVI-7288 in Marburg and AVI-7100 in influenza are in active clinical development. AVI -7537 in Ebola is no longer in clinical development as a result of the October 2012 notice we received from the DoD, terminating the program for the development of AVI-7537 for the convenience of the government due to funding constraints. The rest of our product candidates are in preclinical development, with eteplirsen being our most advanced product candidate with a confirmatory study expected to be initiated later this year. We expect that much of our effort and many of our expenditures over the next several years will be devoted to development 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. With current resources, we may be restricted or delayed in our ability to develop these and other clinical and preclinical product candidates.

Our ability to commercialize any of our product candidates, including eteplirsen, depends on first receiving required regulatory approvals. It is possible that our product candidates, including eteplirsen, may never receive regulatory approval, including any designations that would expedite the review or approval process for various reasons, including: any failure to meet the applicable regulatory requirements to obtain regulatory approval for any of our product candidates including any failure to conduct studies with FDA approved designs, file an NDA prior to or in the time-frame suggested by the FDA, demonstrate the safety and effectiveness for any of our product candidates, lack of funding, changes in the regulatory landscape, new scientific developments, including the results for clinical trials of competitor drugs, and the FDA s interpretation and analysis of such developments in connection with our product candidates, manufacturing or other reasons. If we are unable to obtain regulatory approval for any of our current product candidates, it could delay or eliminate any potential product commercialization and product revenue for our Company.

Even if a product candidate receives regulatory approval, the resulting product may not gain market acceptance among physicians, patients, healthcare payers and the medical community. 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:

establishment and demonstration of clinical efficacy and safety and acceptance of the same by the medical community;

cost-effectiveness of the product;

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the availability of adequate reimbursement by third parties, including governmental payers such as the Medicare and Medicaid programs, managed care organizations, and private health insurers;

the product s potential advantage over alternative treatment methods;

whether the product can be produced in commercial quantities and at acceptable costs;

marketing and distribution support for the product; and

any exclusivities applicable to the product; and

if we are unable to develop and commercialize any of our product candidates, if development is delayed or if sales revenue from any product candidate that receives marketing approval is insufficient, we may never reach sustained profitability

We have been granted orphan drug status for certain of our product candidates, but there can be no guarantee that we will be able to prevent third parties from developing and commercializing products that are competitive to these product candidates.

To date we have been granted orphan status under the Orphan Drug Act by the FDA for: two of our product candidates in DMD (including eteplirsen), AVI-6002 and AVI-7537 for the treatment of Ebola virus and AVI-6003 and AVI-7288 for the treatment of Marburg virus. Generally, product candidates granted orphan status are provided with seven years of marketing exclusivity by the FDA upon NDA approval, meaning the FDA will generally not approve applications for product candidates that contain the same active ingredient and are labeled for the same orphan indication. Even if we are the first to obtain marketing exclusivity through an approval of an orphan product in the United States, there are limited circumstances under which a later product from a competitor 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.

To date we have been granted orphan drug medicinal product designations in the European Union for our lead drug candidate, eteplirsen, and AVI-5038 for the treatment of DMD. 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. Pediatric product candidates may be eligible for an additional two years of marketing exclusivity. Although we may have drug candidates that have or may obtain orphan drug exclusivity in Europe, the orphan designation and associated exclusivity period may be modified for several reasons, including the designation criteria may have significantly changed since market authorization of the orphan product, (e.g., product profitability exceeds the criteria for orphan drug designation), there are production or supply problems with 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 have been 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 drug status for any of our products 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 designation. Moreover, 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, respectively, for the same indication as any of our drug candidates for which we plan to file for orphan 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 State or the European Union. 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.

If we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates, our ability to generate revenue will be materially impaired and our business will not be successful.

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by state authorities and the FDA in the United States and other regulatory authorities in other countries, with regulations differing from country to country. Marketing of our product candidates in the United States or foreign countries is not permitted until we obtain the required approvals from the FDA or other applicable foreign regulatory authorities. Obtaining marketing approval is generally a lengthy, expensive and uncertain process in the United States and other countries and approval is not assured for any of our product candidates.

Further, the FDA and other foreign regulatory agencies have substantial discretion in the approval process, and the determination of when or whether regulatory approval, of any type, will be granted for any product candidate we develop. In this regard, even if we believe data collected from clinical trials of our product candidates are promising and our Chemistry, Manufacturing and Controls (CMC) and related manufacturing processes are satisfactory, the FDA or foreign authorities may disagree with our interpretations and determine such data is not sufficient to accept our application or support approval. Furthermore, the FDA or other foreign regulatory agencies may approve a product candidate for fewer indications than requested or may grant approval subject to the performance of post-approval or confirmatory studies for a product candidate. Similarly, the FDA or other foreign regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates.

In addition, changes in (i) regulatory requirements, (ii) FDA interpretations of scientific developments in diseases targeted by us or our competitors or data and information we submit to the FDA about our product candidates and (iii) FDA guidance and requirements for approval may occur and we may need to amend clinical trial protocols or our approval strategies, including the timing of our expected filings with the FDA, to reflect or address these changes. These changes or amendments may require us to resubmit our clinical trial protocols to institutional review boards (IRBs) or the FDA for review, which may impact the costs, timing or successful completion of a clinical trial, NDA filing and regulatory approval for a product candidate. A therapeutic commercial product utilizing our RNA-based technologies and the manufacturing techniques necessary to produce them at commercial scale have never been approved or validated by any regulatory authority and the FDA may require the Company to make or develop changes in its protocols that will take time and resources, sometimes not estimable, to develop. In addition, the FDA may not approve of the trial designs, protocols and regulatory filings or the timing of the same that we use for our product candidates, including for the confirmatory clinical study and NDA filing for eteplirsen, and the FDA may decline to approve our products on this basis. Changes in the approval process for our product candidates, including those described above may require additional studies or require the Company to address additional issues or requests that were not originally planned, budgeted for or expected by the Company. Other factors may also impact our ability to

obtain or impact the timing of approval for our product candidates, affect the receptiveness of regulators to our compounds, protocols or otherwise impact the regulatory process for our drug candidates including regulatory or other setbacks faced by third parties developing similar compounds or developing drug candidates targeting the same, similar or related diseases as those targeted by our drug candidates. For example, in one of our meetings with the FDA, based on developments in natural history studies and other data from clinical trials for investigational drugs developed by other companies, the FDA indicated it had considerable doubt about the use of dystrophin as a biomarker and questioned the efficacy support provided by the 6MWT in our ongoing open label study. Our exon-skipping therapy uses antisense oligonucleotides and, to date, only one antisense oligonucleotide has been approved by the FDA for systemic use and no product

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using antisense oligonucleotides for systemic use has been approved for sale in the European Union. We cannot be certain that our technology will meet applicable safety and efficacy standards or that we will be able to comply with all the requirements, including those relating to trial design or protocols for studies for our product candidates, of regulatory authorities. Due to these factors, among others, our current product candidates or any of our other future 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 any of our product candidates.

We continue to work with the FDA in our pursuit to obtain FDA approval of eteplirsen. On April 21, 2014, based on recent FDA feedback, we announced our plans to submit an NDA for eteplirsen for the treatment of DMD by the end of the 2014. Additionally, we are working with the FDA to start several additional clinical studies with eteplirsen in exon 51 amenable genotypes and a placebo-controlled study with one or more of our follow-on DMD exon-skipping drug candidates by the end of 2014. Although we believe we are in a position to accomplish our plans within the timeframes indicated, there may be delays in executing them. Furthermore, there can be no assurance that any submission or application will be accepted and filed by the FDA (*e.g.*, refusal to file) or that any expedited or regular development, review or approval will be granted on a timely basis, or at all. As part of its guidance to the Company, the FDA identified additional safety and efficacy data that could be supportive of an NDA filing decision. If the Company is unable to obtain such additional data, the data is negative, or it is inconsistent with prior data it may impact the Company s decision to submit an NDA and/or result in an FDA decision not to file any such submission. The FDA or other foreign authorities could also request additional information or meetings with us or require us to conduct further studies or CMC-related work (*e.g.*, a complete response letter) prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development, review or approval for eteplirsen or any of our other product candidates would result in a longer time period for commercialization of such product candidate, could potentially increase the cost of development of such product candidate, could have a material adverse effect on our financial condition and could harm our competitive position in the marketplace.

Additionally, even if we receive regulatory approval for our product candidates, we will be subject to ongoing FDA obligations and oversight, including adverse event reporting requirements, marketing restrictions and, potentially, other post-marketing obligations such as confirmatory studies, all of which may result in significant expense and limit our ability to commercialize any such products. For example, the FDA has indicated that the confirmatory studies for eteplirsen should be underway at the time of approval. If the studies fail to demonstrate the safety and efficacy of eteplirsen, or follow-on candidates, the FDA may make additional requests that we may not be able to comply with for financial or other reasons and there may be delays in or we may not be able to market or commercialize eteplirsen or some or all of our current DMD product candidates. The FDA s policies may also change and additional government regulations may be enacted that could further restrict or regulate post-approval activities. We cannot predict the likelihood, nature or extent of adverse government regulation that may arise from future legislation or administrative action, either in the United States, or abroad. If we are not able to maintain regulatory compliance, we may be subject to civil and criminal penalties, we may not be permitted to market our products and our business could suffer.

Any delay in, or failure to, receive or maintain regulatory approval for any of our product candidates could harm our business and prevent us from ever generating meaningful revenues or achieving profitability. We will also need to obtain regulatory approval from regulatory authorities in foreign countries to market our product candidates in those countries. We have not submitted an application for regulatory approval to market our product candidates in any foreign jurisdiction. Approval by one regulatory authority does not ensure approval by regulatory authorities in other jurisdictions. If we fail to obtain approvals from foreign jurisdictions, the geographic market for our product candidates would be limited.

Our preclinical and clinical trials may fail to demonstrate acceptable levels of safety and efficacy 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 or efficacy to obtain regulatory approvals.

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 at weeks 24 and 48, respectively, and results reported for weeks 62, 74, 84, 96 and 120 supported stabilization of disease progression, we cannot assure you that data from the ongoing open label extension study will continue to be positive through the study periods. Furthermore, success in preclinical and early clinical trials, such as Study 202, do not ensure that the subsequent confirmatory trials we plan to initiate this year will be successful nor does it predict final results. If the data from the confirmatory studies for eteplirsen do not demonstrate the safety and efficacy data required by regulatory authorities for an NDA filing or approval, we may need to continue working with regulatory authorities on the design and subsequent execution of any further studies we plan to conduct or that may be required for the approval of eteplirsen or our other DMD product candidates. Additional requirements for regulatory approval could increase our costs and delay submissions, studies and commercialization of eteplirsen and continued development of our other DMD product candidates. We may not be able to, or it may be difficult 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 NDA filings, and even if we conform to any guidance regulatory authorities provide, it does not guarantee receipt of marketing approval, even if we believe our preclinical, clinical and confirmatory trials are successful.

We currently rely on certain third-party manufacturers and other third parties for production of our product candidates and our dependence on these manufacturers may impair the advancement of our research and development programs and the development of our product candidates.

We do not currently have the internal ability to manufacture our product candidates in the quantities that we need to conduct our clinical trials and we rely upon a limited number of manufacturers to supply our product candidates and the components of our drug substances. We also need to rely on manufacturers for the production of our product candidates to support our research and development programs. In addition, we rely on other third parties to perform additional steps in the manufacturing process, including filling and labeling of vials and storage of our product candidates. For the foreseeable future, we expect to continue to rely on contract manufacturers and other third parties to produce product candidates and their components, fill vials, and store sufficient quantities of our product candidates for research and development programs, clinical trials and potential commercial supply. For each of our eteplirsen, Marburg and other development programs, based on limited capacity for our specialized manufacturing needs we have had to enter into limited or, at times, non-exclusive sole-source agreements with multinational manufacturing firms for the production of the active pharmaceutical ingredients (APIs) for eteplirsen, Marburg and other therapeutics. There are a limited number of companies that can produce APIs in the quantities and with the quality and purity that we require. Establishing a relationship with alternative suppliers can be a lengthy process and might cause delays in our development efforts. If we are required to seek alternative supply arrangements, the resulting delays and potential inability to find a suitable replacement could materially and adversely impact our business.

Our product candidates require precise, high-quality manufacturing. 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. Contract drug manufacturers often encounter difficulties involving

production yields, quality control and quality assurance and shortages of qualified personnel. If our contract manufacturers or other third parties fail to deliver our product candidates for our research and development programs, clinical use or potential commercial supply on a timely basis, with sufficient quality, and at commercially reasonable prices, and we fail to find replacement manufacturers or to develop our own manufacturing capabilities, we may be required to delay or suspend clinical trials, research and development programs, commercial supply or otherwise discontinue development and production of our product candidates. In addition, we

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currently depend on certain third-party vendors, which in some cases may be sole sources, for the supply of raw materials used to produce our product candidates. If the third-party suppliers were to cease production or otherwise fail to supply us with sufficient quantities of quality raw materials and we are unable to contract on acceptable terms for these raw materials with alternative suppliers, if any, our ability to have our product candidates manufactured in sufficient quantities for preclinical testing, clinical trials, and potential commercial use would be adversely affected.

We do not yet have all of the agreements necessary for the supply of APIs and raw materials for the production of any of our product candidates in quantities sufficient for the potential commercial demand and we may not be able to establish or maintain sufficient commercial manufacturing arrangements on commercially reasonable terms. Securing commercial quantities of our product candidates and their components from contract manufacturers will require us to commit significant capital and resources. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties. In addition, contract manufacturers have a limited number of facilities in which our product candidates can be produced and any interruption of the development or operation of those facilities due to events such as order delays for equipment or materials, equipment malfunction or failure or damage to the 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.

Our contract manufacturers are required to produce our product candidates under current Good Manufacturing Practice (cGMP) conditions in order to meet acceptable standards for our clinical trials. If such standards change, the ability of contract manufacturers to produce our product candidates on the schedule we require for our clinical trials may be affected. In addition, contract manufacturers may not perform their agreements with us or may discontinue their business before the time required by us to successfully produce and market our product candidates. We and our contract manufacturers are subject to periodic unannounced inspection 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. 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 approval by the FDA and corresponding state and foreign authorities, prevent the import and/or export of our products, cause us to lose revenue, or cause our products to be recalled or withdrawn.

We may not be able to successfully scale-up manufacturing of our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing resulting approved drug products, if any.

To date, our product candidates have been manufactured in small quantities for preclinical studies and early stage clinical trials. As we prepare for later stage clinical trials in eteplirsen and potential commercialization, we are working to increase the scale of production of our drug product in 2014. During 2014, we will also continue to increase API production capacity to provide the drug product needed for the additional trials we plan to conduct for eteplirsen, the placebo controlled study planned for one of our follow-on exons and any subsequent commercialization, on an accelerated or other pathway. In order to conduct larger or late-stage scale clinical trials for a product candidate and supply sufficient quantities of the resulting drug product and its components, if that product candidate is approved for sale, we will need to manufacture it in larger quantities. We may not be able to successfully increase the manufacturing capacity for any of our product candidates, whether in collaboration with third-party manufacturers or on our own, in a manner that is timely, safe, compliant with cGMP conditions or other applicable legal or regulatory standards or cost-effective or at all. If a contract manufacturer makes improvements in the manufacturing process for our product candidates, we may not own, or may have to share, the intellectual property rights to those improvements. Significant scale-up of manufacturing may require additional processes, technologies

and validation studies, which are costly, may not be successful and which the FDA must review and approve. In addition, quality issues may arise during those scale-up activities because of the inherent properties of a product candidate itself or of a product candidate in combination with other components added during the manufacturing and packaging process, or during shipping and storage of the finished product or active pharmaceutical ingredients. If we are unable to successfully scale-up manufacture of any of our product candidates in sufficient quality and quantity, the development of that product candidate and regulatory approval or commercial launch for any resulting drug products may be delayed or there may be a shortage in supply, which could significantly harm our business.

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 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.

We rely on U.S. government contracts to support certain research and development programs and for substantially all of our current revenue. If the U.S. government fails to fund such programs on a timely basis or at all, or such contracts are terminated, the results of our operations could be materially and adversely affected.

We rely on U.S. government contracts and awards to fund and support certain development programs, including the Marburg program which accounts for substantially all of our current revenue. The funding of U.S. government programs is subject to Congressional appropriations. Congress generally appropriates funds on a fiscal year basis even though a program may extend over several fiscal years, as is the case with our DoD contract for the development of our Marburg product candidate. Consequently, programs are often only partially funded initially and additional funds are committed only as Congress makes further appropriations. If appropriations for one of our programs become unavailable, or are reduced or delayed, our contracts may be terminated or adjusted by the U.S. government, which could have a negative impact on our future revenue under such contract or subcontract. From time to time, when a formal appropriation bill has not been signed into law before the end of the U.S. government s fiscal year, Congress may pass a continuing resolution that authorizes agencies of the U.S. government to continue to operate, generally at the same funding levels from the prior year, but does not authorize new spending initiatives, during a certain period. During such a period, or until the regular appropriation bills are passed, delays can occur in U.S. government procurement due to lack of funding and such delays can affect our operations during the period of delay. The DoD operated under such a continuing resolution for the U.S. government s fiscal year 2013. Additionally, on March 1, 2013, a sequestration went into effect which implements across-the-board cuts to U.S. government agencies, totaling $1.2 trillion over 10 years. These cuts are to be split 50-50 between domestic and defense discretionary spending. The DoD had to make $47 billion in cuts before September 30, 2013. While Congress struck a two-year budget deal to provide sequester relief in 2014 and 2015, the deal lessens the cuts but does not cancel the sequestration. These and other potential budget cuts by the U.S. government as well as the effects of U.S. government shutdowns could have widespread ramifications including on the DoD s procurement and research and development programs. Sequestration may result in a reduction of funds available for new procurements, but existing contracts may also be reduced in scope, terminated, or partially terminated. The Department of Health and Human Services (DHHS) Special Reserve Fund, a $5.6 billion advanced appropriation to be used over 10 years to purchase medical countermeasures, expired at the end of the 2013 fiscal year. Going forward, Congress plans to replenish the Special Reserve Fund through the annual appropriations process introducing uncertainty with respect to availability of funding from year to year. As a result, the viability of the DHHS and its agencies as a partner and potential customer is uncertain.

In addition, U.S. government contracts generally also permit the U.S. government to terminate or renegotiate the contract, in whole or in part, without prior notice, at the U.S. government s convenience or for default based on performance. From time to time, we receive communications from the U.S. government regarding our performance, including requests for us to provide additional information and/or take certain steps to remedy noted deficiencies.

While we work closely with our contacts at the U.S. government and believe we can adequately address issues raised through such communications, there is no guarantee that we will be able to adequately respond to all requests or remedy all deficiencies cited. If one of our contracts is terminated for convenience, we would generally be entitled to payments for our allowable costs and would receive some allowance for profit on the work performed. If one of our contracts is terminated for default, we would generally be entitled to payments for our work that has been completed to that point. A termination arising out of our default could expose us to liability and have a negative impact on our ability to obtain future contracts. Furthermore, if we fail to satisfy certain performance or deliverable requirements or to adhere to development timelines, revenues associated with the satisfaction of such requirements or timelines may be delayed or may not be realized.

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The termination of one or more of these U.S. government contracts, whether due to lack of funding, for convenience, for our failure to perform, or otherwise, or the occurrence of delays or product failures in connection with one or more of these contracts, could negatively impact our financial condition. For example, on October 2, 2012, we received notice from the DoD that the program for the development of our Ebola product candidate was terminated for the convenience of the U.S. government due to funding constraints. We had previously received a stop-work order for the Ebola program which was in effect from August 2, 2012 through the termination on October 2, 2012. If the U.S. government terminates or reduces the Marburg development program or contract, our business could be materially and adversely affected. Furthermore, we can give no assurance that we would be able to procure new U.S. government contracts to offset the revenue lost as a result of termination of any of our existing contracts. Even if our Marburg contract is not terminated and is completed, there is no assurance that we will receive future U.S. government contracts.

Even if we successfully complete development of our Marburg and influenza product candidates, the major, if not only, potential purchaser is the U.S. government. The lack of a commercial market makes us reliant upon the U.S. government to determine and communicate the market for biodefense countermeasures and U.S. government purchasing is subject to evolving threat assessments and shifting political priorities, which exacerbate market uncertainties. Within the DoD, the war fighter has evolving requirements including but not limited to those related to route of exposure, time to treat, and manufacturing demands. The FDA s requirements under the Animal Rule are also evolving which may result in additional studies being needed to characterize appropriate animal models. It is unclear whether funding will continue to be available to address evolving DoD and FDA requirements, and until future studies are completed, it is unclear whether our product candidates will successfully meet these requirements. If they do not, the DoD may choose to terminate the contract. Additionally, manufacturing demands may be such as to require enhancements to our manufacturing infrastructure, which DoD may not be able to fund through our existing research and development contract. With respect to the civilian sector, Marburg and influenza viruses are among the top public health threats, yet the broader demand for our product candidates remains uncertain.

This expected dependence on U.S. government purchases presents additional challenges, since the U.S. government is incentivized to negotiate prices for countermeasures to just above their marginal cost of production, which would severely limit our profit potential. If companies resist low prices, the U.S. government can, in extreme cases, threaten compulsory licensing or purchase patent-breaching generics.

Our U.S. government contracts may be terminated and we may be liable for penalties under a variety of procurement rules and regulations and changes in government regulations or practices could adversely affect our profitability, cash balances or growth prospects.

We must comply with laws and regulations relating to the formation, administration and performance of U.S. government contracts, which affect how we do business with our customers. Such laws and regulations may potentially impose added costs on our business and our failure to comply with them may lead to penalties and the termination of our U.S. government contracts. Some significant regulations that affect us include:

- the Federal Acquisition Regulation and supplements, which regulate the formation, administration and performance of U.S. government contracts;

- the Truth in Negotiations Act, which requires certification and disclosure of cost and pricing data in connection with contract negotiations; and

the Cost Accounting Standards, which impose accounting requirements that govern our right to reimbursement under certain cost-based government contracts.

Our contracts with the DoD are subject to periodic review and investigation. If such a review or investigation identifies improper or illegal activities, we may be subject to civil or criminal penalties or administrative sanctions, including the termination of contracts, forfeiture of profits, the triggering of price reduction clauses, suspension of payments, fines and suspension or debarment from doing business with U.S. government agencies. We could also suffer harm to our reputation if allegations of impropriety were made against us, which would impair our ability to win awards of contracts in the future or receive renewals of existing contracts.

In addition, U.S. government agencies routinely audit and review their contractors performance on contracts, cost structure, pricing practices and compliance with applicable laws, regulations and standards. They also review the adequacy of, and a contractor s compliance with, its internal control systems and policies, including the contractor s purchasing, property, estimating, compensation and management information systems. Such audits may result in adjustments to our contract costs, and any costs found to be improperly allocated will not be reimbursed. We have recorded contract revenues for the periods presented in this report based upon costs we expect to realize upon final audit; however, we do not know the outcome of any future audits and adjustments and, if future audit adjustments exceed our estimates, our results of operations could be adversely affected. Additionally, we may be required to enter into agreements and subcontracts with third parties, including suppliers, consultants and other third party contractors in order to satisfy our contractual obligations pursuant to our agreements with the DoD. Any such agreement also has to be compliant with the terms of our government grants. Negotiating and entering into such arrangements can be time-consuming and we may not be able to reach agreement with such third parties. Any delay or inability to enter into such arrangements or entering into such arrangements in a manner that is non-compliant with the terms of our grants, may result in violations of our contracts with the DoD.

Clinical trials for our product candidates are expensive and time consuming, may take longer than we expect or may not be completed at all, and their outcomes are uncertain.

We have completed a Phase Ib/II clinical trial for eteplirsen in the UK and announced results in October 2010, which were published in The Lancet in July 2011. We have also completed a U.S.-based Phase IIb placebo-controlled trial in eteplirsen and announced results in April 2012. Following completion of this study, we initiated an open label extension study with the same participants from the original Phase IIb placebo-controlled trial and announced 48-week results on October 3, 2012, 62-week results on December 7, 2012, 74-week results on April 5, 2013, 84-week results on June 19, 2013, 96-week results on September 26, 2013 and 120-week results on January 15, 2014. We expect to commence additional confirmatory trials of eteplirsen and a placebo controlled study on one of our follow-on exon product candidates in 2014 based on feedback from the FDA. Each of our clinical trials requires the investment of substantial planning, expense and time, and the timing of the commencement, continuation and completion of these clinical trials may be subject to significant delays relating to various causes including new positions, issues and requests made by the FDA based on scientific developments and data from other drugs being developed by other companies for the treatment of diseases similar to or related to those targeted by our product candidates. Participant enrollment is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, the eligibility criteria for the trial, the existence of competing clinical trials and the availability of alternative or new treatments.

We depend on medical institutions and clinical research organizations (CROs), to conduct our clinical trials in compliance with Good Clinical Practice (GCP) and to the extent they fail to enroll participants for our clinical trials, fail to conduct the study to GCP standards or are delayed for a significant time in the execution of our trials, including achieving full enrollment, we may be affected by increased costs, program delays or both, which may harm our business. In addition, we have in the past conducted clinical trials in foreign countries and may do so again in the future, which may subject us to further delays and expenses as a result of increased drug shipment costs, additional

regulatory requirements and the engagement of foreign CROs, as well as expose us to risks associated with less experienced clinical investigators who are unknown to the FDA, and different standards of medical care. Foreign currency transactions insofar as changes in the relative value of the U.S. dollar to the foreign currency where the trial is being conducted may impact our actual costs. In addition, for some programs, such as DMD and Marburg infection, there are currently no approved drugs to compare against and an agreement about how to measure efficacy has yet to be reached with the FDA and then demonstrated.

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Clinical trials must be conducted in accordance with FDA or other applicable foreign government guidelines and are subject to oversight by the FDA, other foreign governmental agencies and IRBs at the medical institutions where the clinical trials are conducted. The FDA or other foreign governmental agencies or we ourselves could delay, suspend or halt our clinical trials of a product candidate for numerous reasons, including:

- scientific developments and data available for investigational drugs being developed by third parties for the treatment of the same, similar or related diseases to those targeted by our product candidates;
- deficiencies in the trial design;
- deficiencies in the conduct of the clinical trial including failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols;
- deficiencies in the clinical trial operations or trial sites resulting in the imposition of a clinical hold;
- the product candidate may have unforeseen adverse side effects, including fatalities, or a determination may be made that a clinical trial presents unacceptable health risks;
- the methods and time required to determine whether the product candidate is effective may take longer than expected;
- fatalities or other adverse events arising during a clinical trial that may not be related to clinical trial treatments;
- the product candidate may appear to be no more effective than current therapies;
- the quality or stability of the product candidate may fail to conform to acceptable standards;
- our inability to produce or obtain sufficient quantities of the product candidate to complete the trials;
- our inability to reach agreement on acceptable terms with prospective CROs and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- our inability to obtain IRB approval to conduct a clinical trial at a prospective site;

our inability to obtain regulatory approval to conduct a clinical trial;

lack of adequate funding to continue the clinical trial, including the occurrence of unforeseen costs due to enrollment delays, requirements to conduct additional trials and studies and increased expenses associated with the services of our CROs and other third parties;

our inability to recruit and enroll individuals to participate in clinical trials for reasons including lack of patients or competition from other clinical trial programs for the same or similar indications; or

our inability to retain participants who have initiated a clinical trial but may be prone to withdraw due to side effects from the therapy, lack of efficacy or personal issues, or who are lost to further follow-up.

In addition, we may experience significant setbacks in advanced clinical trials, even after promising results in earlier trials, such as unexpected adverse events that occur when our product candidates are combined with other therapies and drugs or given to larger populations, which often occur in later-stage clinical trials. In addition, clinical results are frequently susceptible to varying interpretations by regulatory authorities that may delay, limit or prevent regulatory approvals. Also, patient advocacy groups and parents of trial participants may demand additional clinical trials or continued access to therapies even if our interpretation of clinical results received thus far leads us to determine that additional clinical trials or continued access are unwarranted. Any disagreement with patient advocacy groups or parents of trial participants may require management s time and attention and may result in legal proceedings being instituted against us, which could be expensive, time-consuming and distracting, and may result in a delay of the program. Negative interpretation of our data by us or regulatory authorities or inconclusive results or adverse medical events, including participant fatalities that may be attributable to our product candidates during a clinical trial may necessitate that it be redesigned, repeated or terminated. Further, some of our clinical trials may be overseen by an independent data and safety monitoring board (DSMB) and a DSMB may determine to delay or suspend one or more of these trials due to safety or futility findings based on events occurring during a clinical trial. Any such delay, suspension, termination or request to repeat or redesign a trial could increase our costs and prevent or significantly delay our ability to commercialize our product candidates.

The Animal Rule is a seldom-used approach to seeking approval of a new drug and our infectious disease program may not meet the requirements for this path to regulatory approval.

Clinical trials cannot be used to assess the efficacy of most biodefense countermeasures against rare and lethal pathogens due to ethical considerations and the relative infrequency of naturally occurring cases. In the United States, we plan to develop the therapeutic product candidate to treat Marburg virus using the Animal Rule regulatory mechanism. Pursuant to the Animal Rule, the sponsor of a drug product must demonstrate efficacy in animal models and safety in humans. There is no guarantee that the FDA will agree to this approach to the development of our infectious disease product candidate, considering that no validated animal model has been established as predicting human outcomes in the prevention or treatment of any filovirus disease. Animal models represent, at best, a rough approximation of efficacy in humans, and, as such, countermeasures developed using animal models will be untested until their use in humans during an emergency. We have yet to demonstrate the predictive value of our animal studies to the FDA s satisfaction. If we fail to do so, we will have to demonstrate efficacy of AVI-7288 through adequate well-controlled trials in humans in order to obtain regulatory approval of this product in the United States, which, if possible given that known Marburg outbreaks have only occurred sporadically in Africa, will greatly add to the time and expense required to commercialize this product. Furthermore, the Animal Rule mechanism has been used only rarely and questions remain regarding the FDA s interpretation and implementation. Of the few times this mechanism has been used as the basis of approval, most of the products approved built upon existing indications with human data

to support efficacy previously approved products which had considerable prior human experience. We do not have any experience successfully navigating this approach to drug approval. Even if the Animal Rule represents a viable approach to seeking approval of AVI-7288, it may present challenges for gaining final regulatory approval for this product candidate, including an extended timeline to approval and less predictable study requirements. In addition, the FDA would require post-marketing human efficacy studies if the countermeasure is used in humans, which would most likely be in the aftermath of a bioterrorist attack. The ability to reliably perform efficacy clinical trials in the midst of a national crisis is uncertain.

The timing and conduct of animal studies may be further constrained given that filoviruses are classified for use only in BSL-4 laboratories. There are limited laboratories and staff world-wide that can work with these live viruses and companies will be competing for the limited availability of this critical infrastructure to test their countermeasures. Furthermore, we anticipate limits in conforming to Good Laboratory Practice (GLP) requirements given the requirement for BSL-4 containment.

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

We incurred an operating loss of $25.1 million for the three months ended March 31, 2014. Our accumulated deficit was $571.5 million as of March 31, 2014. Substantially all of our revenues to date have been derived from research and development contracts with the DoD. We have not yet generated any material revenue from product sales and have 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 and acquired in-process research and development resulting from two acquisitions. We anticipate that our expenses will increase substantially if and as we:

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

We will likely 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. 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. 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 or to conduct clinical trials and to market our product candidates. Other than pre-clinical collaborations with academic/research institutions and government entities for the development of additional exon-skipping product candidates for the treatment of DMD and 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 other than that with the U.S. government. Such relationships may require us to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

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 and 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.

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

Our RNA-based 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. We are conducting toxicology, pharmacology, pharmacokinetics and other preclinical studies and, although we have conducted Phase I clinical trials for AVI-6003 (we are now pursuing development of AVI-7288, one of the two component oligomers in AVI-6003) and AVI-7100 and conducted a Phase IIb clinical trial in eteplirsen, additional preclinical studies may be required for these product candidates and before other product candidates enter human clinical trials. In addition, preclinical models to study participant toxicity and activity of compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease and there may be substantially different results in clinical trials from the results obtained in preclinical studies. Any failures or setbacks in utilizing our PMO-based technology, including adverse effects resulting from the use of this technology 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.

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If we fail to retain our key personnel or are unable to attract and retain additional qualified personnel, our future growth, ability to perform our U.S. government contracts 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-based therapeutics and related technologies and personnel with experience overseeing compliance with and execution of the terms of our U.S. government contracts. The loss of the services of any one of the principal members of our managerial, scientific or government contract compliance staff may prevent us from achieving our business objectives.

The competition for qualified personnel in the biotechnology field and for qualified personnel with government contracting experience 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 managerial, scientific and government contract compliance staff. In certain instances, we may also need to expand or replace our workforce and our management ranks. We face intense competition for qualified individuals from numerous pharmaceutical and biotechnology companies, as well as academic and other research institutions. If we are unable to attract, assimilate or retain such key personnel, our ability to advance our proprietary programs and perform our U.S. government contracts would be adversely affected. Any failure to perform under our U.S. government contracts could result in a termination of the agreement, which would harm our business.

If we are unable to manage our growth effectively, execute our business strategy and effectively implement compliance controls and systems, the trading price of our common stock could decline. Although we did not have a material error in our financial statements, we have identified a material weakness in our internal control over financial reporting as of December 31, 2013. Any ongoing failure to establish and maintain effective internal control over financial reporting could adversely affect investor confidence in our reported financial information.

We are a development stage company and 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 this in a timely and efficient manner could adversely affect our operations.

For example, although there was no material error in our 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. A detailed description of this material weakness is provided in Item 9A, Controls and Procedures of our annual report on Form 10-K filed earlier this year. During the first quarter of 2014, we designed and implemented controls to adequately review and consider the recognition and measurement of new significant research and development contracts. Our controls were designed to ensure that such contracts are timely reviewed by our accounting personnel with the requisite accounting knowledge, skills, and experience deemed necessary to perform such a review. However, we cannot assure you 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 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 revenues, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our 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. The risks we face on the intellectual property front include the following:

- our patent rights might be challenged, invalidated, or circumvented, or otherwise might not provide any competitive advantage;
- 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; and
- jurisdictions other than the U.S. might have less restrictive patent laws than the U.S., giving foreign competitors the ability to exploit these laws to create, develop, and market competing products.

In addition, the United States Patent and Trademark Office (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.

Additionally, the U.S. Supreme Court has issued decisions, the full impact of which are 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

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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, might have or obtain patents that limit, interfere with, or eliminate our ability to make, use, and sell our product candidates in important commercial markets.

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

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

Any of these events could substantially harm our potential earnings, financial condition, and operations. Prosensa Holding B.V. (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, 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. The Opposition Division decision if maintained at the appeals level could have a substantial effect on our businesses and leaves open the possibility that Prosensa or other parties that have rights to such patent could assert that our drug eteplirsen infringes on such patent. The timing and outcome of appeal cannot be predicted or determined as of the date of this report. We are also aware of existing patent claims Prosensa is pursuing in the United States, and others that it has or is pursuing, in other jurisdictions, including Japan, that where granted may provide the basis for Prosensa or other parties to assert that commercialization of eteplirsen would infringe on such claims.

The DMD patent landscape is continually evolving and multiple parties, 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 those 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 program.

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, Isis Pharmaceuticals and Santaris Pharma A/S (Santaris) share a focus on RNA-based drug discovery and development. Competitors with respect to our exon-skipping DMD program, or eteplirsen, include Prosensa and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs.

Although Prosensa/ GSK announced in 2013 that the primary endpoint for their lead DMD drug candidate was not met, we may still 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.

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 clinical trial 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, and the sale of any approved products in the future, may expose us to liability claims. 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

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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 conditions. We expect that our operations will be affected by other new environmental and health and workplace safety laws on an ongoing basis, and although we cannot predict the ultimate impact of any such new laws, they may impose greater compliance costs or result in increased risks or penalties, which could harm our business.

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

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

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

In the ordinary course of business we may, and in some cases have, become involved in lawsuits and other disputes such as securities claims, intellectual property challenges and employee matters. We may not prevail in claims made against us in such disputes. The outcome of such lawsuits and disputes is inherently uncertain. We do not believe that any of the current lawsuits or disputes faced by us is likely to have a material adverse effect on our business, financial condition or results of operations, but the resolution of such lawsuits or disputes could have a material adverse effect on our results for that period. Also, lawsuits or claims brought against us in the future could have a material adverse effect 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, have been historically volatile. For example, during the first quarter of 2014, our stock traded from a low of $17.50 per share to a high of $31.28 per share. Additionally, on November 12, 2013 our stock price decreased 64% on the same day that we made an announcement regarding an unexpected FDA communication that the FDA considered an NDA application for eteplirsen premature despite earlier communications indicating it was open to an NDA application. Furthermore, on April 21, 2014 our stock price increased 39% on the same date that we announced that, based on FDA feedback, we planned to file an NDA for the approval of eteplirsen by the end of 2014. 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:

- the timing of our filings with regulatory authorities and regulatory decisions and developments including the probability of a decision by the FDA to review eteplirsen on an expedited or normal pathway, if at all;

positive or negative results 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 government regulations or requirements by regulatory in the approval process;

developments concerning proprietary rights, including patents and litigation matters;

public concern relating to the commercial value or safety of any of our products;

financing, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;

comments by securities analysts;

litigation; or

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

Broad market and industry factors may seriously affect the market price of companies 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.

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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. Some of these fluctuations may be very pronounced such as in the case of the impact to our net loss as a result of our warrant offerings in January and August 2009 of which warrants for an aggregate of 0.6 million shares remain outstanding and exercisable as of March 31, 2014. Each of these warrants is classified as a derivative liability and accordingly, the fair value of the warrants is recorded on our consolidated balance sheet as a liability, and such fair value is adjusted at each financial reporting date with the adjustment to fair value reflected in our consolidated statement of operations and comprehensive loss. For example, for the three months ended March 31, 2014, the impact of the change in fair value of these warrants resulted in a $3.3 million loss in our unaudited condensed consolidated statement of operations and comprehensive loss. The fair value of the warrants is determined using the Black-Scholes-Merton option-pricing model. Fluctuations in the assumptions and factors used in the Black-Scholes-Merton option-pricing model can result in adjustments to the fair value of the warrants reflected on our balance sheet and, therefore, our statement of operations and comprehensive loss. Due to the classification of such warrants and other factors, results of operations are difficult to forecast, and period-to-period comparisons of our operating results may not be predictive of future performance. Additionally, our operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Specifically, a change in the timing of activities performed in support of our U.S. government research contracts could either accelerate or defer anticipated revenue from period to period. Likewise, our research and development expenses may experience fluctuations as a result of the timing of activities performed in support of our U.S. government research contracts and 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 warrants, and we expect to issue additional stock awards and shares of common stock in the future. Exercise of these awards, and sales of shares will dilute the interests of existing security holders and may depress the price of our common stock.

As of March 31, 2014, there were 38.0 million shares of common stock outstanding, outstanding awards to purchase 5.4 million shares of common stock under various incentive stock plans, and outstanding warrants to purchase up to 0.6 million shares of common stock. Additionally, as of March 31, 2014, 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 available for issuance under our 2013 Employee Stock Purchase Plan and 0.6 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, 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.

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Item 6. Exhibits.

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

SIGNATURES

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

SAREPTA THERAPEUTICS, INC.

Date: May 8, 2014

By: /s/ CHRISTOPHER GARABEDIAN
Christopher Garabedian
President and Chief Executive Officer

Date: May 8, 2014

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

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EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.1	Offer Letter dated January 6, 2014 by and between Sarepta Therapeutics, Inc. and Arthur Krieg, M.D.					X
31.1	Certification of the Company s President and Chief Executive Officer, Christopher Garabedian, 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 President and Chief Executive Officer, Christopher Garabedian, 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.

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