

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

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2013

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)

Oregon
(State or other jurisdiction of
incorporation or organization)

93-0797222
(I.R.S. Employer
Identification No.)

215 First Street Suite 7, Cambridge,
Massachusetts

(Address of principal executive offices)

02142
(Zip Code)

Registrant's telephone number, including area code: (857) 242-3700

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

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

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

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

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

Common Stock with \$0.0001 par value
(Class)

31,909,542
(Outstanding as of April 30, 2013)

[Table of Contents](#)

SAREPTA THERAPEUTICS, INC.
FORM 10-Q
INDEX

	<u>Page</u>
PART I — FINANCIAL INFORMATION	
Item 1. Financial Statements	3
Unaudited Condensed Consolidated Balance Sheets — March 31, 2013 and December 31, 2012	3
Unaudited Condensed Consolidated Statements of Operations and Comprehensive Loss — Three Months Ended March 31, 2013 and 2012 and from July 22, 1980 (Inception) through March 31, 2013	4
Unaudited Condensed Consolidated Statements of Cash Flows — Three Months Ended March 31, 2013 and 2012 and from July 22, 1980 (Inception) through March 31, 2013	5
Notes to Unaudited Condensed Consolidated Financial Statements	6
Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations	13
Item 3. Quantitative and Qualitative Disclosures about Market Risk	21
Item 4. Controls and Procedures	22
PART II — OTHER INFORMATION	
Item 1. Legal Proceedings	22
Item 1A. Risk Factors	22
Item 2. Unregistered Sales of Equity Securities and Use of Proceeds	37
Item 3. Defaults Upon Senior Securities	37
Item 4. Mine Safety Disclosures	37
Item 5. Other Information	37
Item 6. Exhibits	38
Signatures	40
Exhibits	41

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)

	<u>March 31,</u> <u>2013</u>	<u>December 31,</u> <u>2012</u>
Assets		
Current Assets:		
Cash and cash equivalents	\$ 167,919	\$ 187,661
Accounts receivable	5,652	4,713
Other current assets	3,023	1,534
Total Current Assets	176,594	193,908
Restricted investments	7,250	—
Property and equipment, net of accumulated depreciation and amortization of \$17,024 and \$16,708	3,088	3,397
Patent costs, net of accumulated amortization of \$1,534 and \$2,626	4,883	4,913
Other assets	1,525	2,775
Total Assets	<u>\$ 193,340</u>	<u>\$ 204,993</u>
Liabilities and Shareholders' Equity		
Current Liabilities:		
Accounts payable	\$ 7,073	\$ 7,532
Accrued employee compensation	1,520	2,741
Long-term debt, current portion	90	89
Warrant liability	91,077	65,193
Deferred revenue	3,884	3,304
Other liabilities	15	27
Total Current Liabilities	103,659	78,886
Commitments and Contingencies		
Long-term debt	1,645	1,668
Other long-term liabilities	755	760
Total Liabilities	106,059	81,314
Shareholders' 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; 31,888,949 and 31,703,817 issued and outstanding	3	3
Additional paid-in capital	560,613	554,927
Deficit accumulated during the development stage	(473,335)	(431,251)
Total Shareholders' Equity	87,281	123,679
Total Liabilities and Shareholders' Equity	<u>\$ 193,340</u>	<u>\$ 204,993</u>

See accompanying notes to condensed consolidated financial statements.

[Table of Contents](#)

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

	<u>Three months ended March 31,</u>		<u>July 22, 1980</u>
	<u>2013</u>	<u>2012</u>	<u>(Inception) through</u>
			<u>March 31, 2013</u>
Revenues from license fees, grants and research contracts	\$ 4,474	\$ 11,212	\$ 178,022
Operating expenses:			
Research and development	13,762	14,805	399,430
General and administrative	6,127	3,281	125,214
Acquired in-process research and development	—	—	29,461
Operating loss	<u>(15,415)</u>	<u>(6,874)</u>	<u>(376,083)</u>
Other non-operating income (loss):			
Interest income and other, net	237	96	9,760
Loss on change in warrant liability	(26,906)	(10,926)	(93,874)
Realized gain on sale of short-term securities — available-for-sale	—	—	3,863
Write-down of short-term securities — available-for-sale	—	—	(17,001)
	<u>(26,669)</u>	<u>(10,830)</u>	<u>(97,252)</u>
Net loss	<u>\$ (42,084)</u>	<u>\$ (17,704)</u>	<u>\$ (473,335)</u>
Other comprehensive income (loss):			
Write-down of short-term securities — available-for-sale	—	—	17,001
Realized gain on sale of short-term securities — available-for-sale	—	—	(3,863)
Unrealized loss on short-term securities — available-for-sale	—	—	(13,138)
	<u>—</u>	<u>—</u>	<u>—</u>
Comprehensive loss	<u>\$ (42,084)</u>	<u>\$ (17,704)</u>	<u>\$ (473,335)</u>
Net loss per share — basic and diluted	<u>\$ (1.32)</u>	<u>\$ (0.78)</u>	
Weighted average number of common shares outstanding for computing basic and diluted net loss per share	<u>31,813</u>	<u>22,624</u>	

See accompanying notes to condensed consolidated financial statements.

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

	<u>Three months ended March 31,</u>		<u>For the Period</u>
	<u>2013</u>	<u>2012</u>	<u>July 22, 1980</u>
			<u>(Inception) through</u>
			<u>March 31, 2013</u>
Cash flows from operating activities:			
Net loss	\$ (42,084)	\$ (17,704)	\$ (473,335)
Adjustments to reconcile net loss to net cash flows used in operating activities:			
Depreciation and amortization	451	353	22,421
Loss on disposal of assets	196	65	2,832
Realized gain on sale of short-term securities — available-for-sale	—	—	(3,863)
Write-down of short-term securities — available-for-sale	—	—	17,001
Impairment charge on real estate owned	—	—	1,445
Stock-based compensation	1,671	708	33,744
Acquired in-process research and development	—	—	29,461
Increase in warrant liability	26,906	10,926	93,874
Net increase in accounts receivable and other assets	(1,178)	(1,830)	(9,939)
Net increase (decrease) in accounts payable, accrued employee compensation, and other liabilities	(955)	(1,567)	11,458
Net cash used in operating activities	<u>(14,993)</u>	<u>(9,049)</u>	<u>(274,901)</u>
Cash flows from investing activities:			
Purchase of restricted investments	(7,250)	—	(7,250)
Purchase of property and equipment	(9)	(9)	(19,996)
Patent costs	(461)	(253)	(10,990)
Purchase of marketable securities	—	—	(112,993)
Sale of marketable securities	—	—	117,724
Acquisition costs	—	—	(2,389)
Net cash used in investing activities	<u>(7,720)</u>	<u>(262)</u>	<u>(35,894)</u>
Cash flows from financing activities:			
Proceeds from sale of common stock, warrants, and partnership units, net of offering costs, and exercise of options and warrants	2,993	1	479,552
Repayments of long-term debt	(22)	(21)	(452)
Other financing activities, net	—	—	(386)
Net cash provided by (used in) financing activities	<u>2,971</u>	<u>(20)</u>	<u>478,714</u>
Increase (decrease) in cash and cash equivalents	(19,742)	(9,331)	167,919
Cash and cash equivalents:			
Beginning of period	<u>187,661</u>	<u>39,904</u>	<u>—</u>
End of period	<u>\$167,919</u>	<u>\$ 30,573</u>	<u>\$ 167,919</u>
Supplemental Disclosure of Cash Flow Information			
Cash paid during the period for interest	\$ 22	\$ 22	\$ 597
Supplemental Schedule of Noncash Investing Activities and Financing Activities:			
Short-term securities — available-for-sale received in connection with private offerings	\$ —	\$ —	\$ 17,897
Issuance of common stock in satisfaction of warrants and other liabilities	\$ 1,022	\$ —	\$ 33,856
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 condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

1. ORGANIZATION 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 the Company’s 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 drug candidates, including its lead product candidate, eteplirsen, which is currently in a Phase IIb clinical trial. 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 and expects to continue to receive significant financial support from U.S. government research contracts.

The accompanying unaudited condensed consolidated financial statements reflect the accounts of Sarepta and its consolidated subsidiaries. The accompanying unaudited condensed consolidated balance sheet data as of December 31, 2012 was derived from audited financial statements not included in this report. The accompanying unaudited condensed consolidated financial statements were prepared in conformity with accounting principles generally accepted in the United States of America (GAAP) and the rules and regulations of the U.S. Securities and Exchange Commission (SEC) pertaining to interim financial statements. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements.

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 accompanying unaudited condensed consolidated financial statements reflect all adjustments that are, in the opinion of management, necessary for a fair presentation of the financial position, results of operations and cash flows for the interim periods. The accompanying unaudited condensed consolidated financial statements should be read in conjunction with the financial statements and the notes thereto included in the Company’s annual report on Form 10-K for the year ended December 31, 2012. The results of operations for the interim periods presented are not necessarily indicative of the results to be expected for the full year.

Since its inception in 1980, the Company has incurred losses of \$473.3 million, substantially all of which resulted from expenditures related to research and development, general and administrative charges and losses on change in warrant valuation partially offset by revenue generated from research contracts with and grants primarily from the U.S. Department of Defense (DoD). As of March 31, 2013, the Company has completed all of its contracts with the DoD except for the July 2010 contract and the August 2012 contract for the development of therapeutics against the Marburg virus. In November 2012 the Company also entered into an agreement with the European Commission (EC) Health Innovation for development and study related activities for a Duchenne muscular dystrophy (DMD) therapeutic for which minimal revenues have been earned to date. The Company has not generated any material revenue from product sales to date, and there can be no assurance that revenues from product sales will be achieved. Moreover, even if the Company does achieve revenue from product sales, the Company is likely to continue to incur operating losses in the near term.

As of March 31, 2013, the Company has \$167.9 million of cash and cash equivalents which the Company believes is more than sufficient to fund operations for the next twelve months. Should the Company’s funding from the DoD 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, seeking additional government contracts, and from establishing collaborations or licensing its technology to other companies.

Reverse Split of Common Stock

In July 2012, the Company effected a one-for-six reverse split of its issued and outstanding shares of common stock. Following the reverse split, the total number of shares outstanding was proportionately reduced and any outstanding options, warrants and rights were adjusted accordingly. All share and per share amounts have been adjusted to reflect the retroactive application of the reverse stock split.

[Table of Contents](#)

Estimates and Uncertainties

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting period. Actual results could differ from those estimates.

Commitments and Contingencies

As of the date of this report, the Company is not a party to any material legal proceedings with respect to itself, its subsidiaries, or any of its material properties. 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 employment, intellectual property, and effects from the use of therapeutics utilizing its technology, professional services or others. It is impossible to predict whether any resulting liability would have a material adverse effect on the Company's financial position, results of operations or cash flows.

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. To meet the requirement of the letters of credit, the Company purchased \$7.3 million in certificates of deposit with April 2014 maturity dates during the three months ended March 31, 2013. The Company has recorded this \$7.3 million as restricted investments in the condensed consolidated balance sheet as of March 31, 2013.

2. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of common shares outstanding. Diluted net loss per share is computed by dividing net loss by the weighted-average number of common shares and dilutive common stock equivalent shares outstanding. Given that the Company was 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.

	Three Months Ended March 31,	
	2013	2012
	(in thousands, except per share amounts)	
Net loss	\$ (42,084)	\$ (17,704)
Weighted-average number of shares of common stock and common stock equivalents outstanding:		
Weighted-average number of common shares outstanding for computing basic earnings per share	31,813	22,624
Dilutive effect of outstanding warrants and stock awards after application of the treasury stock method*	—	—
Weighted-average number of common shares outstanding for computing diluted earnings per share	31,813	22,624
Net loss per share — basic and diluted	\$ (1.32)	\$ (0.78)

[Table of Contents](#)

* Warrants, stock options, restricted stock units (RSUs) and stock appreciation rights (SARs) to purchase approximately 5,997,000 and 7,159,000 shares of common stock were excluded from the net loss per share calculation for the three months ended March 31, 2013 and 2012, respectively, as their effect would have been anti-dilutive.

3. FAIR VALUE MEASUREMENTS

The Company measures at fair value certain financial assets and liabilities in accordance with a hierarchy of valuation techniques based on whether the inputs to those valuation techniques are observable or unobservable. Observable inputs reflect market data obtained from independent sources, while unobservable inputs reflect the Company's market assumptions. There are three levels of inputs that may be used to measure fair-value:

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

The Company's assets and liabilities measured at fair value on a recurring basis consisted of the following as of the date indicated:

	Fair Value Measurement as of March 31, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Restricted investments	\$ 7,250	\$ 7,250	\$ —	\$ —
Total assets	\$ 7,250	\$ 7,250	\$ —	\$ —

	Fair Value Measurement as of December 31, 2012			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Restricted investments	\$ —	\$ —	\$ —	\$ —
Total assets	\$ —	\$ —	\$ —	\$ —

	Fair Value Measurement as of March 31, 2013			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants*	\$ 91,077	\$ —	\$ —	\$ 91,077
Total liabilities	\$ 91,077	\$ —	\$ —	\$ 91,077

	Fair Value Measurement as of December 31, 2012			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Warrants*	\$ 65,193	\$ —	\$ —	\$ 65,193
Total liabilities	\$ 65,193	\$ —	\$ —	\$ 65,193

* See Note 5 for additional information related to the determination of fair value of warrants and a reconciliation of changes in fair value.

The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents, accounts receivable, 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 because of similar characteristics to other debt instruments with comparable risk.

4. ACCOUNTS RECEIVABLE

Accounts receivable are generally stated at invoiced amount and do not bear interest. Because the accounts receivable are primarily from the DoD and historically no amounts have been written off, an allowance for doubtful accounts receivable is not considered necessary. The accounts receivable balance included \$4.7 million and \$3.2 million of DoD receivables that were unbilled at March 31, 2013 and December 31, 2012, respectively.

5. WARRANTS

The Company has periodically issued warrants in connection with certain common stock offerings. The warrants issued in January 2009 and August 2009 are classified as liabilities as opposed to equity because their settlement terms 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. All other warrants issued by the Company were recorded as additional paid-in-capital and no further adjustments are made.

The outstanding warrants classified as liabilities are recorded at fair value on the condensed consolidated balance sheet 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 liability" in the condensed consolidated statement of operations and comprehensive loss. 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 used in the model. The following reflects the weighted-average assumptions for each of the periods indicated:

	Three Months Ended March 31,	
	2013	2012
Risk-free interest rate	0.1%	0.2%-0.3%
Expected dividend yield	0%	0%
Expected lives	0.8-1.4 years	0.7-2.4 years
Expected volatility (1)	99.4%-110.7%	65.1%-82.4%
Shares underlying warrants classified as liabilities	3,093,676	4,824,827
Market value of stock at beginning of year	\$ 25.80	\$ 4.50
Market value of stock at end of period	\$ 36.95	\$ 9.24

- (1) For the three month period ended March 31, 2013, expected volatility has been 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. Prior to January 1, 2013, expected volatility has been estimated using calculated volatility of the Company's common stock over a historical period commensurate with the expected term of the option.

A reconciliation of the change in value of the Company's warrants recorded as liabilities for the three months ended March 31, 2013 and 2012 is as follows:

	Three Months Ended March 31,	
	2013 (in thousands)	2012 (in thousands)
Balance at beginning of period	\$ 65,193	\$ 5,446
Increase in value of warrants	26,906	10,926
Reclassification to shareholders' equity upon exercise of warrants	(1,022)	—
Balance at end of period	\$ 91,077	\$ 16,372

[Table of Contents](#)

For the three months ended March 31, 2013, 33,942 warrants were exercised at a weighted average exercise price of \$8.16, generating proceeds of \$0.3 million. For the three months ended March 31, 2012, no warrants were exercised.

The following table summarizes the outstanding warrants at March 31, 2013.

<u>Issue Date</u>	<u>Exercise Price</u>	<u>Outstanding Warrants at March 31, 2013</u>	<u>Expiration Date</u>	<u>Weighted Average Remaining Contractual Life (Years)</u>	<u>Exercisable Warrants</u>
1/30/2009	\$ 6.96	1,819,567	7/30/2014	1.3	1,819,567
1/30/2009	\$ 8.70	3,912	1/30/2014	0.8	3,912
8/25/2009	\$ 10.68	1,270,197	8/31/2014	1.4	1,270,197
		<u>3,093,676</u>			<u>3,093,676</u>

6. EQUITY FINANCING

In January 2013, the Company sold approximately 87,000 shares of common stock through its outstanding At-The-Market (ATM) offering. The sale generated \$2.1 million in net proceeds and fully exhausted the sales of stock available under the ATM sales agreement.

7. CONTRACT REVENUE

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

The following table sets forth the revenue for each of the Company's contracts with the U.S. and E.U. governments and other revenue for the three months ended March 31, 2013 and 2012.

	<u>Three Months Ended March 31,</u>	
	<u>2013</u>	<u>2012</u>
	<u>(in thousands)</u>	
July 2010 Contract (<i>Ebola and Marburg IV</i>)	\$2,614	\$11,163
August 2012 Contract (<i>Intramuscular</i>)	1,806	—
November 2012 SKIP-NMD Agreement (<i>DMD</i>)	54	—
Other Agreements	—	49
Total	\$ 4,474	\$ 11,212

U.S. Government Contracts

As of March 31, 2013, the Company had completed all of its contracts with the DoD except for the Marburg portion of the July 2010 contract for the development of therapeutics against Ebola and Marburg viruses and the August 2012 contract for intramuscular (IM) administration of AVI-7288, the Company's candidate against the Marburg virus.

July 2010 Contract (Ebola and Marburg Intravenous administration)

On July 14, 2010, the Company was awarded a DoD contract managed by the Joint Project Manager Transformational Medical Technologies (JPM-TMT) Project Management Office, a component of the Joint Program Executive Office for Chemical and Biological Defense, for the advanced development of the Company's 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 U.S. Food and Drug Administration (FDA) to proceed with a single oligomer from AVI-6003, AVI-7288, as the lead product candidate against Marburg virus infection.

[Table of Contents](#)

On August 2, 2012, the Company received a stop-work order related to the Ebola virus portion of the contract and, on October 2, 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 the DoD exercises its options for all segments. Activities under the first segment began in July 2010 and include Phase I studies in healthy volunteers as well as preclinical studies.

After completion of the first segment, and each successive segment, the DoD has the option to proceed to the next segment. If the 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.

August 2012 Contract (Intramuscular administration)

On August 29, 2012, the Company was awarded a contract from the DoD, which is also managed by the JPM-TMT. The contract was awarded for approximately \$3.9 million to evaluate the feasibility of an IM route of administration using AVI-7288, the Company's candidate for treatment of Marburg virus. The period of performance of this contract is scheduled to conclude in the second half of 2013.

European Union Agreement

In November 2012, the Company entered into an agreement for a collaborative research project partially funded by the EC Health Innovation. The agreement provides for reimbursement of costs of 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 quarter ended March 31, 2013, the Company received \$635,000 in advance payments and recognized \$54,000 of these payments as revenue. Deferred revenue related to the agreement as of March 31, 2013 was \$581,000. The remaining balance of deferred revenue relates to the Company's sponsored research agreement with Charley's Fund.

8. STOCK 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, RSUs and SARs.

Stock-based compensation costs are based on the fair value calculated utilizing the Black-Scholes-Merton option pricing model on the date of grant. The fair value of stock awards, with consideration given to estimated forfeitures, is amortized as compensation expense on a straight-line basis over the vesting period of the grants.

As of March 31, 2013, 916,903 shares of common stock remain available for future grant under the 2011 Equity Incentive Plan. During the three months ended March 31, 2013, there were no grants, exercises or cancellations for restricted stock awards, RSUs or SARs except for 262 RSUs which were canceled during the period.

Stock Options

In general, stock options granted prior to December 31, 2010 vest over a three year period, with one-third of the underlying shares vesting on each anniversary of grant, and have a ten year term. Beginning in January 2011, stock options granted generally vest over a four year period, with one-fourth of the underlying shares vesting on the first anniversary of the grant and the remaining underlying shares vesting pro-ratably on a monthly basis thereafter, such that the underlying shares will be fully vested on the fourth anniversary of the grant.

[Table of Contents](#)

A summary of the Company's stock option activity with respect to the three months ended March 31, 2013 follows:

<u>Stock Options</u>	<u>Underlying Shares</u>	<u>Weighted Average Exercise Price</u>	<u>Weighted Average Remaining Contractual Term</u>	<u>Aggregate Intrinsic Value</u>
Outstanding at December 31, 2012	2,522,522	\$ 11.76		
Granted	242,320	28.76		
Exercised	(64,390)	8.97		
Canceled	(5,774)	14.83		
Outstanding at March 31, 2013	<u>2,694,678</u>	<u>\$ 13.35</u>	<u>8.3</u>	<u>\$ 63,877,000</u>
Vested at March 31, 2013 and expected to vest	<u>2,502,875</u>	<u>\$ 13.24</u>	<u>8.3</u>	<u>\$ 59,634,000</u>
Exercisable at March 31, 2013	<u>656,805</u>	<u>\$ 12.36</u>	<u>6.8</u>	<u>\$ 16,428,000</u>

The weighted-average fair value per share of stock-based awards granted to employees during the three months ended March 31, 2013 and 2012 was \$18.08 and \$5.46, respectively. During the three months ended March 31, 2013 and 2012, the total intrinsic value of stock options exercised was \$1,402,000 and \$280 respectively, and the total grant date fair value of stock options that vested was \$624,000 and \$1,942,000, respectively.

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

	<u>Three Months Ended March 31,</u>	
	<u>2013</u>	<u>2012</u>
Risk-free interest rate	0.7%	1.1%
Expected dividend yield	0%	0%
Expected lives	4.9 years	5.3 years
Expected volatility	80.0%	79.7%

Stock-based Compensation Expense

A summary of the stock-based compensation expense, including stock options, restricted stock, RSUs, and SARs recognized in the condensed consolidated statements of operations and comprehensive loss is as follows:

	<u>Three Months Ended</u>	
	<u>March 31, 2013</u>	<u>March 31, 2012</u>
	<u>(in thousands)</u>	
Research and development	\$ 530	\$ 253
General and administrative	1,141	455
Total	<u>\$ 1,671</u>	<u>\$ 708</u>

As of March 31, 2013, there was \$19.8 million of unrecognized compensation cost related to non-vested share-based compensation arrangements outstanding including stock options, restricted stock, RSUs, and SARs. These costs are expected to be recognized over a weighted-average period of 3.0 years.

9. INCOME TAXES

At December 31, 2012, the Company had net deferred tax assets of approximately \$114.1 million. The net deferred tax assets are primarily composed of U.S. federal and state tax net operating loss carryforwards, U.S. federal and state research and development credit carryforwards and share-based compensation expense. 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

[Table of Contents](#)

offset its net deferred tax asset. Additionally, the Internal Revenue Code rules could limit the future use of its net operating loss and research and development credit carryforwards to offset future taxable income based on ownership changes and the value of the Company's stock.

10. 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 is expected to be completed in 2013. During the quarter ended March 31, 2013, the Company recorded a restructuring charge of \$264,000 to research and development expense and \$198,000 to general and administrative expense. All transition costs are expected to be paid in 2013.

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

	Three Months Ending March 31, 2013 <small>(in thousands)</small>
Balance at December 31, 2012	\$ 185
Restructuring charges	462
Payments	(201)
Balance at March 31, 2013	<u>\$ 446</u>

11. RECENT ACCOUNTING PRONOUNCEMENTS

In February 2013, the Financial Accounting Standards Board (FASB) issued new guidance which requires disclosure of significant amounts reclassified out of accumulated other comprehensive income by component and their corresponding effect on the respective line items of net income. This guidance was adopted by the Company in fiscal year 2013. The adoption of this guidance did not have an impact on the Company's unaudited condensed consolidated financial statements.

12. SUBSEQUENT EVENTS

On April 10, 2013, the Company and the University of Western Australia (UWA) entered into an agreement under which the existing Exclusive License Agreement by and between the Company and UWA was amended and restated (the "Amended and Restated UWA Agreement").

Under the terms of the Amended and Restated UWA Agreement, UWA granted the Company an exclusive license to certain UWA intellectual property rights relating to the use of antisense oligonucleotides to induce exon skipping for the treatment of certain muscular dystrophies. The Amended and Restated UWA Agreement provides that the Company must use commercially reasonable efforts to develop, commercialize and market certain products covered by the licensed intellectual property rights ("Products"). Under the Amended and Restated UWA Agreement, the UWA is eligible to receive up to \$7.1 million in upfront and development milestone payments, as well as specified royalties (in the low single-digit percentage range) as a percentage of net sales of Products.

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, 2012 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;

[Table of Contents](#)

- the results of our research and development efforts and the efficacy of our PMO-based chemistries and other RNA-based technology;
- our expectations regarding our ability to become a leading developer and marketer of RNA-based therapeutics;
- our expectations regarding the results of preclinical and clinical testing of our product candidates;
- 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 completing planning for a pivotal clinical trial for eteplirsen by the end of 2013 and commencing dosing in this trial in early 2014;
- our ability to submit IND filings for DMD candidates beyond eteplirsen;
- the receipt of any required approval from the FDA or other regulatory approval for our products;
- the potential for our product candidates to qualify for accelerated approval, as breakthrough therapies or as orphan drugs;
- the potential for any filings or applications by us for accelerated approval or other designations to be accepted or granted by the FDA, in addition to the potential and timing for supplemental informational submissions and meetings with the FDA;
- the effect of regulation by the FDA and other agencies;
- our intention to introduce new products;
- our expectations regarding the markets, pricing or reimbursement for our products;
- acceptance of our products, if introduced, in the marketplace;
- the impact of competitive products, product development, commercialization and technological difficulties;
- our expectations regarding our ability to commercialize eteplirsen with a relatively small sales force, if eteplirsen is approved for commercial sale;
- our expectations regarding partnering opportunities and other strategic transactions;
- our ability to increase the scale of our manufacturing to provide our product to patients in larger scale clinical trials or in potential commercial quantities;
- our ability to operate our business without infringing the intellectual property rights of others;
- 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 estimates regarding our future revenues, research and development expenses, other expenses, payments to third parties and changes in staffing levels;

[Table of Contents](#)

- our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and statements about our future capital needs;
- our expectations about funding from the 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.

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 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 which are primarily funded and supported by the DoD, and leveraging our highly-differentiated, 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 unmet medical need. Last year, 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 that is expected to be completed in late 2013 with the same participants from the original Phase IIb placebo controlled trial. We anticipate initiating a pivotal clinical trial for eteplirsen by the end of 2013 and commencing dosing in this trial in early 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 phosphorodiamidate-linked morpholino oligomer, or 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.

We were incorporated in the State of Oregon on July 22, 1980. Our executive office is located at 215 First Street, Suite 7 Cambridge, MA 02142 and our telephone number is (857) 242-3700. Our common stock trades on The NASDAQ Global Market under the symbol “SRPT.” On July 12, 2012, our common stock began trading on The NASDAQ Global Market on a split-adjusted basis following a one-for-six reverse stock split that was effective on July 11, 2012. Unless otherwise noted, all share amounts, share prices and exercise prices included throughout this report give effect to the July 2012 one-for-six reverse stock split.

Since our inception in 1980, we have incurred losses of \$473.3 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 research contracts with and grants primarily from the DoD. As of March 31,

[Table of Contents](#)

2013, we have completed all of our contracts with the DoD except for the July 2010 contract and the August 2012 contract for the development of therapeutics against the Marburg virus. In November 2012 we also entered into an agreement with the EC Health Innovation for development and study related activities for a DMD therapeutic for which minimal revenues have been earned to date. We have not generated any material revenue from product sales to date, and there can be no assurance that revenues 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, 2013, we had \$167.9 million of cash and cash equivalents which we believe is more than sufficient to fund operations for the next twelve months. Should our funding from the DoD 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, seeking additional government contracts, and by establishing collaborations or licensing our technology to other companies.

Government Contracts

We recognize revenue from government research contracts during the period in which the related expenditures are incurred and present these revenues and related expenses gross in the condensed consolidated financial statements. In the periods presented, substantially all of the revenues generated by us was derived from research contracts with the DoD. As of March 31, 2013, we had completed all of our contracts with the DoD except for the Marburg portion of the July 2010 agreement for the development of therapeutics against Ebola and Marburg viruses and the August 2012 contract for IM administration of AVI-7288, our candidate against the Marburg virus.

The following table sets forth the revenue from each of our contracts with the U.S. and E.U. governments and other revenue for the three months ended March 31, 2013 and 2012.

	Three Months Ended March 31,	
	2013	2012
	(in thousands)	
July 2010 Contract (<i>Ebola and Marburg IV</i>)	\$ 2,614	\$ 11,163
August 2012 Contract (<i>Intramuscular</i>)	1,806	—
November 2012 SKIP-NMD Agreement (<i>DMD</i>)	54	—
Other Agreements	—	49
Total	\$ 4,474	\$ 11,212

July 2010 Contract (Ebola and Marburg Intravenous administration)

On July 14, 2010, we were awarded the DoD contract managed by the JPM-TMT Project Management Office 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-6003, AVI-7288, as the lead product candidate against Marburg virus infection.

On August 2, 2012, we received a stop-work order related to the Ebola virus portion of the contract and, on October 2, 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 Phase I studies in healthy volunteers as well as preclinical studies.

After completion of the first segment, and each successive 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.

[Table of Contents](#)

August 2012 Contract (Intramuscular administration)

On August 29, 2012, we were awarded a contract from the DoD, which is also being managed by the JPM-TMT. The contract was awarded for approximately \$3.9 million to evaluate the feasibility of an IM route of administration using AVI-7288, our candidate for treatment of Marburg virus. The period of performance of this contract is scheduled to conclude in the second half of 2013.

November 2012 SKIP-NMD Agreement (DMD)

In November 2012, we entered into an agreement for a collaborative research project partially funded by the EC 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.

Key Financial Metrics

Revenue

Government Research Contract and Grant Revenue. Substantially all of our revenue is generated from U.S. government research contracts and grants. See Note 7 of the Notes to the Unaudited Condensed Consolidated Financial Statements included elsewhere in this Quarterly Report on Form 10-Q. We recognize revenue from government research contracts and grants during the period in which the related expenses are incurred and present such revenue and related expenses gross in the condensed consolidated financial statements. Government contract revenue is highly dependent on the timing of various activities performed by us and our third party vendors. Changes in the timing of activities performed in support of this contract have, and may in the future, result in unexpected fluctuations in our revenue from period to period. We expect that future revenue generated under our government contracts will continue to be variable as a result of these factors.

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, 2013, we had deferred revenue of \$3.9 million, which represents up-front fees which we will recognize as revenue as we satisfy the outstanding performance obligations.

Expenses

Research and Development. Research and development expense consists of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, and clinical trial and manufacturing costs.

Direct research and development expenses associated with our programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants and other outside 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 expense 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 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 we have anticipated, may fail to receive necessary regulatory approvals, or may prove impracticable to manufacture in commercial quantities at reasonable cost and with acceptable quality.

[Table of Contents](#)

As a result of these uncertainties and the other risks inherent in the drug development process, we cannot determine the duration and 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 and sale 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 expense consists principally of salaries, benefits, stock-based compensation expense, and related costs for personnel in our executive, finance, legal, information technology, business development and human resource functions. Other general and administrative expenses include an allocation of our facility costs and professional fees for legal, consulting and accounting services.

Interest Income and Other, Net. Interest income and other, net, primarily consists of interest on our cash and cash equivalents, interest expense, and rental income. Our cash equivalents consist of money market investments. Interest expense includes interest paid on our mortgage loan related to the Corvallis property, the substantial portion of which we leased in November 2011. Rental income is from subleasing excess space in some of our facilities.

Loss on Change in Warrant Liability. 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; we are not required to expend any cash to settle these liabilities. The fair market value of these warrants was recorded on the balance sheet at issuance and the warrants are marked to market each financial reporting period, with changes in the fair value recorded as a gain or loss in our statement of operations. 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 market valuation primarily due to changes in our stock price. For more information, see Note 5 of the Notes to the Unaudited Condensed Consolidated Financial Statements included elsewhere in this Quarterly Report on Form 10-Q.

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 condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States, or 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 that we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our 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;
- stock-based compensation; and
- accounting for and valuation of warrants classified as liabilities.

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, 2012 filed with the Securities and Exchange Commission, or SEC, on March 15, 2013.

Results of Operations for the Three Months Ended March 31, 2013 and 2012

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

	Three Months Ended March 31,		% Change
	2013	2012	
	(in thousands, except per share amounts)		
Revenue	\$ 4,474	\$ 11,212	(60)%
Operating expenses:			
Research and development	13,762	14,805	(7)%
General and administrative	6,127	3,281	87%
Operating loss	(15,415)	(6,874)	124%
Other income (loss):			
Interest income and other, net	237	96	147%
Loss on change in warrant liability	(26,906)	(10,926)	146%
Net loss	\$ (42,084)	\$ (17,704)	138%
Basic and diluted loss per share	\$ (1.32)	\$ (0.78)	

Revenue

Revenue for the three months ended March 31, 2013 decreased by \$6.7 million, or 60%, compared to the three months ended March 31, 2012. The decrease was primarily due to \$5.2 million from the Ebola portion of the DoD contract due to the August 2012 stop-work order and the subsequent termination for convenience in October 2012 and a decrease of \$3.3 million in revenue associated with the Marburg portion of the DoD contract. The decrease was partially offset by a \$1.8 million increase in revenue on the IM government research contract.

Research and Development Expenses

Research and development expenses for the three months ended March 31, 2013 decreased by \$1.0 million, or 7%, compared to the three months ended March 31, 2012. The decrease was primarily due to \$3.7 million from the Ebola portion of the DoD contract due to the August 2012 stop-work order and the subsequent termination for convenience in October 2012, a decrease of \$2.6 million in costs on the Marburg portion of the DoD contract, and a \$0.5 million decrease in our DMD program costs due to the timing of manufacturing and clinical activities. The decrease was partially offset by a \$4.2 million increase in personnel related costs and costs of proprietary research and \$1.6 million of costs incurred on the IM government research contract.

General and Administrative Expenses

General and administrative expenses for the three months ended March 31, 2013 increased by \$2.8 million, or 87%, compared to the three months ended March 31, 2012. The increase in general and administrative expenses is primarily due to a \$1.9 million increase in personnel costs including \$0.6 million in stock-based compensation and \$0.7 million of additional professional service costs.

Interest (Expense) Income and Other, Net

Interest income (expense) and other, net, for the three months ended March 31, 2013 increased primarily due to interest earned on higher cash and cash equivalents balances compared to the three months ended March 31, 2012.

Loss on Change in Warrant Liability

The change in fair value of our warrant liability for the three months ended March 31, 2013 compared to the three months ended March 31, 2012 was primarily attributable to the change in our stock price. See Note 5 to the Unaudited Condensed Consolidated Financial Statements included elsewhere in this report.

Net Loss

Net loss for the three months ended March 31, 2013 was \$42.1 million, compared to net loss of \$17.7 million for the three months ended March 31, 2012. The increased net loss was primarily due to an increase in operating loss of \$8.5 million and a \$16.0 million increase in non-operating expenses due to the increase in the fair market value of our outstanding warrants. The fair market value of our outstanding warrants is a non-cash expense which is highly impacted by the change in the value of our stock.

[Table of Contents](#)

Liquidity and Capital Resources

At March 31, 2013, cash and cash equivalents were \$167.9 million, compared to \$187.7 million at December 31, 2012. The decrease during the three month period ended March 31, 2013 is due primarily to cash used to operate our business and the purchase of \$7.3 million of restricted investments to secure two letters of credit issued in connection with certain manufacturing agreements. These uses of cash were partially offset by \$3.0 million in net proceeds from the sale of common stock under our ATM sales agreement and warrant and option exercises. Based on the factors described below, we believe that our available cash and cash equivalents is more than sufficient to finance our operations for the next twelve months.

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

Our primary source of revenue is from development of product candidates pursuant to our contracts with the U.S. and E.U. governments. Government funding is subject to the U.S. government's appropriations process and the U.S. government has the right under our contracts with them to terminate such contracts for convenience as was done regarding the Ebola portion of the 2010 Ebola and Marburg contract. If DoD 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 DoD 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, it would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing shareholders could experience substantial dilution.

Historical Trends

	<u>Three Months Ended March 31,</u>	
	<u>2013</u>	<u>2012</u>
	(in thousands)	
Cash provided by (used in):		
Operating activities	\$ (14,993)	\$ (9,049)
Investing activities	(7,720)	(262)
Financing activities	<u>2,971</u>	<u>(20)</u>
Decrease in cash and equivalents	<u>\$ (19,742)</u>	<u>\$ (9,331)</u>

Operating Activities. The increase in the amount of cash used in operating activities of \$5.9 million for the three months ended March 31, 2013 compared to the three months ended March 31, 2012 was primarily due to an increase in operating loss of \$8.5 million driven by lower government contract revenue and higher general and administrative costs partially offset by a reduction in research and development costs. In addition to the increase in operating loss, there was a working capital increase of \$1.3 million, as well as a \$1.0 million increase 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 \$7.5 million for the three months ended March 31, 2013 compared to the three months ended March 31, 2012 was primarily due to the purchase of \$7.3 million

[Table of Contents](#)

of investments in February 2013 to secure two letters of credit issued in connection with certain manufacturing contracts. We also used more cash to fund patent costs in the three months ended March 31, 2013 as compared to the three months ended March 31, 2012.

Financing Activities. The increase in the amount cash from financing activities of \$3.0 million for the three months ended March 31, 2013 compared to the three months ended March 31, 2012 was primarily due to the sale of approximately 87,000 shares of common stock under our ATM sales agreement in January 2013 which generated \$2.1 million in net proceeds and fully exhausted the sales of our stock available under the ATM sales agreement. We also received \$0.9 million in net proceeds from warrant and stock option exercises during the three months ended March 31, 2013 for which we issued approximately 98,000 shares of additional common stock.

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, 2013:

	Payments Due by Period				
	Total	Less Than 1 Year	1-3 Years	3-5 Years	More Than 5 Years
Long-term debt	\$ 1,735	\$ 90	\$ 193	\$ 212	\$ 1,240
Operating leases	13,142	1,973	3,633	2,976	4,560
Purchase obligations (1)	28,988	25,712	3,276	—	—
Total	<u>\$43,865</u>	<u>\$27,775</u>	<u>\$ 7,102</u>	<u>\$ 3,188</u>	<u>\$ 5,800</u>

(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

See Note 11 to the Notes to the Unaudited Condensed Consolidated Financial Statements contained in Part I, Item 1 of this report.

Item 3. Quantitative and Qualitative Disclosures about Market Risk.

We had cash and cash equivalents of \$167.9 million and \$187.7 million at March 31, 2013 and December 31, 2012, respectively. We do not enter into investments for trading or speculative purposes; our cash equivalents are invested in money market accounts. We believe that we do not have any material exposure to changes in the fair value of these assets in the near term due to the short term nature of our cash and cash equivalents. A 0.1% decline in interest rates, occurring January 1, 2013 and sustained throughout the period ended March 31, 2013, would have been inconsequential. Future declines in interest rates, however, would reduce investment income, but are not likely to be a material source of income to us in the foreseeable future.

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, under the supervision and with the participation of our management, including (1) our chief executive officer and (2) our chief financial officer, of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or 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 Securities and Exchange Commission, or 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) 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, 2013, our disclosure controls and procedures were effective.

Changes in Internal Control Over Financial Reporting

There have been no changes in our internal control over financial reporting during the quarter ended March 31, 2013 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings.

As of the date of this report, we are not a party to any material legal proceedings with respect to us, our subsidiaries, or any of our material properties. In the normal course of business, we may from time to time be named as a party to various legal claims, actions and complaints, including matters involving employment, intellectual property, effects from the use of drugs utilizing our technology, or others. It is impossible to predict whether any resulting liability would have a material adverse effect on our financial position, results of operations or cash flows.

Item 1A. Risk Factors.

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 was in active clinical development until August 2012, when we received a stop-work order from the DoD instructing us to cease all work and ordering of supplies in support of the development of this product candidate. On October 2, 2012, we received notice from the DoD that the program for the development of AVI-7537 was terminated for the convenience of the government due to funding constraints. The rest of our product candidates are in preclinical development. 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, and it is possible that we may never receive regulatory approval, including any accelerated approval by the FDA under Subpart H — Accelerated Approval of New Drugs for Serious or Life-Threatening Illnesses or any other designations that will expedite the review or approval process for any of our product candidates, based on an inability to adequately demonstrate the safety and effectiveness of our product candidates, failure to meet other regulatory requirements, lack of funding, changes in the regulatory landscape, manufacturing or other reasons. If we are unable to obtain regulatory approval for any of our product candidates it could delay or eliminate any potential product revenue by delaying or terminating the potential commercialization of our product candidates.

[Table of Contents](#)

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:

- establishment and demonstration of clinical efficacy and safety and acceptance of the same by the medical community;
- cost-effectiveness of the product;
- the availability of adequate reimbursement by third parties, including 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 at acceptable costs;
- marketing and distribution support for the product; and
- any exclusivities applicable to the product.

To date we have been granted orphan status for two of our product candidates in DMD and for AVI-7537 for the treatment of Ebola virus and AVI-7288 for the treatment of Marburg virus. We are not guaranteed to receive orphan status for other product candidates in development or product candidates we may develop in the future. Even though we have received orphan status for some of our product candidates, we would not enjoy orphan drug exclusivity for such product candidates in the event that another company received approval of products with the same active ingredient for the same indication before we receive market approval. 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 a competitor's product receives orphan drug designation for an indication that we are targeting, and such product is approved for commercial sales before our product, regulators may interpret our product to be the same drug as the competing product and could prevent us from selling our product in the applicable territories for the competitor's orphan exclusivity period. Furthermore, pediatric exclusivity only applies if the product has another form of exclusivity.

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.

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 may not be successful.

The research, testing, manufacturing, labeling, approval, selling, marketing and distribution of drug products are subject to extensive regulation by 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 marketing approval from the FDA or other foreign regulatory authorities, and we may never receive regulatory approval for the commercial sale of any of our product candidates. Obtaining marketing approval is a lengthy, expensive and uncertain process and approval is never assured. As of the date of this report, we have not progressed to the point of preparing or filing the applications necessary to gain regulatory approvals.

Further, the FDA and other foreign regulatory authorities 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 the 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, regulatory agencies may approve a product candidate for fewer indications or for a more narrowly defined indication than requested or may grant approval subject to the performance of post-approval studies for a product candidate. Similarly, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates.

[Table of Contents](#)

In addition, changes in regulatory requirements and guidance may occur and we may need to amend clinical trial protocols or other approval strategies to reflect these changes. 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. Changes in our approval strategies may occur that require additional studies that were not originally planned. Other factors may also impact our ability to obtain approval of our product candidates, including, for example, the fact that dystrophin has never been utilized as a surrogate endpoint and 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. 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 product revenue by delaying or terminating the potential commercialization of our product candidates.

For example, we are pursuing FDA approval of eteplirsen, our lead product candidate, and recently reported results from a U.S.-based Phase IIb 12-patient clinical trial for eteplirsen at 30 mg/kg and 50 mg/kg. On April 15 we announced that the FDA provided us with feedback requesting additional information from the existing eteplirsen dataset to inform a decision on the acceptability of this dataset for an NDA filing under the accelerated approval regulatory pathway. This feedback was provided in meeting minutes from Sarepta's End-of-Phase II meeting with the FDA's Division of Neurology. Based on this feedback and information that may be provided to us in future meetings with the FDA, we will evaluate the most appropriate timing and path for pursuing regulatory review and approval of eteplirsen. Any decision will be further informed by a subsequent CMC meeting. There can be no assurance that, after our evaluation, we will decide to pursue or submit an NDA under Subpart H accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval or under another expedited regulatory designation (e.g., breakthrough therapy designation), there can be no assurance that such submission or application will be accepted (e.g., refusal to file) or that any expedited development, review or approval will be granted on a timely basis, or at all. 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 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. 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

[Table of Contents](#)

future clinical trials. While Studies 201 and 202 met their primary endpoints at weeks 24 and 48 respectively, we cannot assure you that data from these studies will be sufficient for regulatory approval or that Study 202 extension study results will continue to be positive through the remaining study period. If these data are not sufficient to demonstrate safety and efficacy to regulators, do not continue to demonstrate safety and efficacy through the remainder of Study 202, or are insufficient to identify a consistently effective dose, we expect we will need to engage in discussions with regulatory authorities about the design and subsequent execution of any further studies which may be required. Regulatory authorities might require more extensive preclinical or clinical trials than anticipated. Such clinical trials might include additional open label “extension studies” for all participants, who have previously received eteplirsen, as well as other participants (e.g., non-ambulatory participants), additional placebo-controlled “pivotal” study or studies, or additional trials before conducting a pivotal trial or trials of the product candidate. Any additional studies required by regulatory authorities would increase our costs and delay commercialization of eteplirsen. Even if we conform to any guidance regulatory authorities provide it does not guarantee receipt of marketing approval, even if we believe our preclinical and clinical trials are successful.

Furthermore, success in preclinical and early clinical trials does not ensure that the ongoing Study 202 and later larger-scale trials will be successful nor does it predict final results. Acceptable results in early trials may not be reproduced in the remainder of the Study 202 extension study or later trials. For example, pivotal trials for eteplirsen will likely involve a larger number of patients to achieve statistical significance, will be expensive and will take a substantial amount of time to complete. As a result, we may conduct lengthy and expensive clinical trials of our product candidates, only to learn that the product candidate is not an effective treatment or is not superior to existing approved therapies, or has an unacceptable safety profile, which could prevent or significantly delay regulatory approval for such product candidate.

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 substance. 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, 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 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 commercial sale 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

[Table of Contents](#)

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 clinical 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 in the first half of 2013 to increase the scale of production of our drug product and planning for mid-scale production by the end of 2013. In 2013, we will also evaluate whether to increase API production capacity to a commercial scale which will depend in significant part on feedback from the FDA and our expectations regarding if and when we would commence a pivotal trial for eteplirsen and any subsequent commercialization. In order to conduct larger or late-stage scale clinical trials for a product candidate and supply sufficient commercial 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 timely or cost-effective manner 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 resulting 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 stability 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 stability, 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 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 would 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 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

[Table of Contents](#)

spending initiatives, during a certain period. During such a period, or until the regular appropriation bills are passed, delays can occur in government procurement due to lack of funding and such delays can affect our operations during the period of delay. Currently the DOD is operating under such a continuing resolution for fiscal year 2013. Additionally, on March 1, 2013, a sequestration went into effect which implements across-the-board cuts to 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 must make \$47 billion in cuts before September 30, 2013. These cuts 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 also existing contracts may also be reduced in scope, terminated, or partially terminated. The 2004 Project BioShield Act which created the Special Reserve Fund for use by the Department of Health and Human Services (DHHS) to purchase countermeasures over 10 years mitigates the uncertainty of the annual appropriations process and sequestration, but the \$5.6 billion advanced appropriation is rapidly depleting and will expire at the end of fiscal year 2013. Thus, the viability of the DHHS and its agencies as a continuing partner and potential customer hinges in part on Congress taking action to replenish the Special Reserve Fund.

In addition, U.S. government contracts generally also permit the government to terminate or renegotiate the contract, in whole or in part, without prior notice, at the 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.

The termination of one or more of these 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 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 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 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 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 specifically related to route of administration and time to treat. Until future studies are completed, it is unclear whether our product candidates will successfully meet these requirements. If it does not, the DoD may choose to terminate the contract. With respect to the civilian sector, Marburg and influenza viruses are among the top chemical, biological, radiological and nuclear threats to national security, yet the DHHS has not defined the civilian requirements, making the broader demand for our product candidates uncertain.

This expected dependence on government purchases presents additional challenges, since the 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, governments can, in extreme cases, threaten compulsory licensing or purchase patent-breaching generics.

[Table of Contents](#)

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 Ib 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 Ib placebo controlled trial and announced 48-week results on October 3, 2012, 62-week results on December 7, 2012 and 74-week result on April 5, 2013. We expect to commence additional trials of eteplirsen and other product candidates in the future. 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. 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 (e.g., 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.

[Table of Contents](#)

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:

- 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 time required to determine whether the product candidate is effective may be 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 or clinical trial sites to participate in clinical trials for reasons including 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 unable to be contracted for 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 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 delay of the program. Negative 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 the 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, 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. Only one novel product has been approved using the Animal Rule. It has thus far been used to extend the indicated use of three 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.

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

We had an operating loss of \$15.4 million for the three months ended March 31, 2013 and incurred an operating loss of \$29.7 million for the year ended December 31, 2012. As of March 31, 2013, our accumulated deficit was \$473.3 million and 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.

[Table of Contents](#)

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 and development 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, it 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 shareholders. To the extent we issue additional equity securities or convertible securities, our existing shareholders could experience substantial dilution in their economic and voting rights. For example, in 2012, we sold approximately 6.9 million shares of our common stock in connection with our September 2012 ATM equity offering program and December 2012 public financing. 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 RNA-based 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 RNA-based 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

[Table of Contents](#)

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.

The relocation of our corporate headquarters and selected research and development activities may create unintended negative consequences, including increased costs and loss of personnel.

We recently moved our corporate headquarters from Bothell, Washington to Cambridge, Massachusetts. We also moved selected research and development activities from Bothell to our existing site in Corvallis, Oregon and will develop additional research and development activities at a future site in Cambridge. We expect the transition will continue through 2013. While we believe the relocation will improve our business operations and enhance our ability to attract and retain industry talent, this relocation may result in the following negative consequences:

- increased costs associated with the closing of our existing facility in Bothell, Washington including the moving of lab equipment to Cambridge and Corvallis;
- increased costs associated with the relocation of personnel, including reimbursement of relocation expenses and cost of living adjustments to base salaries;
- employee turnover due to relocation;
- increased costs associated with retention and/or severance packages for Bothell-based personnel;
- business disruptions resulting from the relocation; and
- increased long-term lease costs.

If any of these consequences occur, the negative impact may outweigh any benefits related to the relocation, which could have an adverse effect on our business.

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 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.

Recent changes in our executive leadership and any similar changes in the future may serve as a significant distraction for our management and employees.

In January 2011, Christopher Garabedian, a member of our board of directors, was hired to serve as our president and chief executive officer. Since the beginning of 2011, there have been a number of changes to our executive leadership team. Most recently, in November 2012, we hired our senior vice president, chief financial officer, Sandesh Mahatme and our senior vice president, general counsel and corporate secretary, David Tyrone Howton. In June 2012, our former senior vice

[Table of Contents](#)

president and chief scientific officer, Peter Linsley, resigned from his employment with us and in February 2012 our former senior vice president and general counsel, Effie Toshav, resigned from her employment with us. Such changes, or any other future changes in our executive leadership, may disrupt our operations as we adjust to the reallocation of responsibilities and assimilate new leadership and, potentially, differing perspectives on our strategic direction. If the transition in executive leadership is not smooth, the resulting disruption could negatively affect our operations and impede our ability to execute our strategic plan.

We may engage in future acquisitions that increase our capital requirements, dilute our shareholders, 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. Any potential acquisitions 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 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 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 United States 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.

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;

[Table of Contents](#)

- redesign products candidates or processes to avoid infringement;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could substantially harm our potential earnings, financial condition, and operations. Prosensa, which is developing competitive pipeline products, has rights to patent claims that, absent a license, may preclude us from commercializing eteplirsen in several jurisdictions. Prosensa has rights to European Patent No. EP 1619249, for example. We opposed this patent in the Opposition Division of the European Patent Office, 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 our drug eteplirsen in the European Union would infringe on such patent. We are also aware of certain patent claims that Prosensa has rights to, and others that it is pursuing, in other jurisdictions, including Japan and the United States, that may provide the basis for Prosensa or other parties that have rights to these claims to assert that commercialization of our drug eteplirsen in such other jurisdictions 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 competing products before or more successfully than we do.

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, competes directly with our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Isis Pharmaceuticals and 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 GSK and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs.

Clinical trials evaluating the systemic administration of the Prosensa/GSK lead DMD drug candidate are currently ongoing, including a placebo-controlled global Phase III clinical trial. Two placebo-controlled Phase II clinical trials, one based in the United States and one based outside the United States have now concluded. The Prosensa/GSK drug candidate may, or may not, prove to be safer or more efficacious than our product candidate and it could gain marketing approval before our product candidate. This might affect our ability to successfully complete a clinical development program or market eteplirsen once approved. This competition may also extend to other exon-skipping drugs for DMD limiting our ability to gain market share.

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;

[Table of Contents](#)

- 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 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 biohazardous 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 cybersecurity 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 liability and our research and development programs and the development of our product candidates could be delayed.

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 2012, our stock has traded from a low of \$3.30 per share to a high of \$45.00 per share. 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:

- our ability to obtain and any decision to pursue Subpart — H accelerated approval for eteplirsen;
- positive or negative results of testing and clinical trials by ourselves, strategic partners, or competitors;
- 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;
- 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, if instituted against us, could result in substantial costs and a diversion of our management's attention and resources.

Provisions of our articles of incorporation, bylaws and Oregon corporate 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 articles 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:

- classification of our board of directors into two classes, with one class elected each year;
- prohibition of cumulative voting of shares in the election of directors;
- prohibition of shareholder actions by less than unanimous written consent;
- express authorization of the board of directors to make, alter or repeal our bylaws;
- advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by shareholders at shareholder meetings; and
- 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 shareholder approval, including rights superior to the rights of the holders of common stock.

[Table of Contents](#)

In addition, the Oregon Control Share Act and Business Combination Act may limit parties that acquire a significant amount of voting shares from exercising control over us for specific periods of time. These provisions could discourage, delay or prevent a transaction involving a change of control, even if doing so would benefit our shareholders. These provisions also could discourage proxy contests and make it more difficult for shareholders to elect directors of their choosing or cause us to take other corporate actions, such as replacing or removing management or members of our board of directors.

We expect our quarterly 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 our warrant offerings in January and August 2009 of which 3.1 million remain outstanding and exercisable as of March 31, 2013. Each of these warrants is classified as a derivative liability and accordingly, the fair value of the warrants is recorded on our condensed 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 condensed consolidated statement of operations. For example, for the three months ended March 31, 2013, the impact of the change in fair value of these warrants resulted in a \$26.9 million charge to our Unaudited Condensed Consolidated Statement of Operations and Comprehensive Loss. The fair value of the warrants is determined using the Black-Scholes-Merton option valuation model. Fluctuations in the assumptions and factors used in the Black-Scholes-Merton model can result in adjustments to the fair value of the warrants reflected on our balance sheet and, therefore, our statement of operations. Due to the classification of such warrants and other factors, quarterly 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 quarterly 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 quarters, 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, 2013, there were 31.9 million shares of common stock outstanding, outstanding awards to purchase 2.9 million shares of common stock under various incentive stock plans and outstanding warrants to purchase up to 3.1 million shares of common stock. Additionally, as of March 31, 2013, there were 0.9 million shares of common stock available for future issuance under our 2011 Equity Incentive Plan. In addition, 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 2011 Equity 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 shareholders 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.

[Table of Contents](#)

On May 9, 2013, in connection with the retention of Michael A. Jacobsen, Vice President, Finance, who is not relocating to our new headquarters in Cambridge, Massachusetts, but is working for the company in the interim to help maintain business continuity during the transition of our new principal executive offices, we entered into a Retention and Severance Benefits Letter Agreement with Mr. Jacobsen (the “Jacobsen Agreement”).

As previously disclosed, on August 23, 2012, Mr. Jacobsen was awarded 7,500 restricted stock units under the company’s 2011 Equity Incentive Plan. Pursuant to the terms of the Jacobsen Agreement, provided that Mr. Jacobsen does not resign, for any reason whatsoever, from his employment with the company on or before May 31, 2013, then the company will terminate Mr. Jacobsen’s employment effective May 31, 2013 (a “Qualifying Termination”) and 100% of the then-unvested restricted stock units will become fully vested on May 31, 2013.

Additionally, as previously disclosed, on April 24, 2012, Mr. Jacobsen was awarded an option to purchase 3,702 shares of the company’s common stock under the company’s 2011 Equity Incentive Plan. Pursuant to the terms of the Jacobsen Agreement, 100% of the then-unvested shares subject to this option will become fully vested if Mr. Jacobsen undergoes a Qualifying Termination.

Also pursuant to the terms of the Jacobsen Agreement, if Mr. Jacobsen undergoes a Qualifying Termination, he will receive: (i) subject to Mr. Jacobsen’s delivery and failure to revoke a general release of claims against the company, \$78,554.66, which amount is approximately equivalent to eight weeks of Mr. Jacobsen’s base salary and a pro-rated 2013 bonus at 100% of target, and (ii) if Mr. Jacobsen elects continuation coverage under COBRA, payment or reimbursement of Mr. Jacobsen’s COBRA healthcare premiums for the one-month period following the Qualifying Termination.

The foregoing description of the terms of the Jacobsen Agreement does not purport to be a complete description and is qualified in its entirety by reference to the Jacobsen Agreement that is filed as Exhibit 10.3 to this Quarterly Report on Form 10-Q.

Item 6. Exhibits.

<u>Exhibit Number</u>	<u>Exhibit Description</u>	<u>Incorporated by Reference to Filings Indicated</u>			<u>Provided Herewith</u>
		<u>Form</u>	<u>File No.</u>	<u>Filing Date</u>	
10.1**	Amended and Restated Exclusive License Agreement dated April 10, 2013 by and among Sarepta Therapeutics, Inc., Sarepta International CV and the University of Western Australia.				X
10.2†	Amended and Restated Executive Employment Agreement dated April 19, 2013 by and between the Company and Christopher Garabedian.				X
10.3†	Retention and Severance Benefits Letter Agreement dated May 9, 2013 by and between the Company and Michael A. Jacobsen.				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

[Table of Contents](#)

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.	
*	In accordance with Rule 406T of Regulation S-T, the information in these exhibits is furnished and deemed not filed or a part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, is deemed not filed for purposes of Section 18 of the Exchange Act of 1934, and otherwise is not subject to liability under these sections and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such filing.	
**	Confidential treatment has been requested for portions of this exhibit.	

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 9, 2013

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

Date: May 9, 2013

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

EXHIBIT INDEX

<u>Exhibit Number</u>	<u>Exhibit Description</u>	<u>Incorporated by Reference to Filings Indicated</u>			
		<u>Form</u>	<u>File No.</u>	<u>Filing Date</u>	<u>Provided Herewith</u>
10.1**	Amended and Restated Exclusive License Agreement dated April 10, 2013 by and among Sarepta Therapeutics, Inc., Sarepta International CV and the University of Western Australia.				X
10.2†	Amended and Restated Executive Employment Agreement dated April 19, 2013 by and between the Company and Christopher Garabedian.				X
10.3†	Retention and Severance Benefits Letter Agreement dated May 9, 2013 by and between the Company and Michael A. Jacobsen.				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.

[Table of Contents](#)

- * In accordance with Rule 406T of Regulation S-T, the information in these exhibits is furnished and deemed not filed or a part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, is deemed not filed for purposes of Section 18 of the Exchange Act of 1934, and otherwise is not subject to liability under these sections and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such filing.
- ** Confidential treatment has been requested for portions of this exhibit.

AMENDED AND RESTATED EXCLUSIVE LICENSE AGREEMENT

THIS AMENDED AND RESTATED EXCLUSIVE LICENSE AGREEMENT (“Agreement”) is effective as of November 24, 2008 (the “Effective Date”), and is restated as of this 10th day of April, 2013 (“Restatement Date”) by and between **THE UNIVERSITY OF WESTERN AUSTRALIA**, a body corporate established pursuant to the provisions of The University of Western Australia Act 1911, with offices at 35 Stirling Highway, Crawley, Western Australia 6009 (“UWA”), on the one hand, and **SAREPTA THERAPEUTICS**, with offices at 245 First Street Suite 1800 Cambridge, MA 02142 USA (“Sarepta”) and **Sarepta International CV** (“Sarepta Netherlands,” and collectively with Sarepta, “Licensee”), on the other hand.

RECITALS

A. UWA owns and is entitled to grant license rights with respect to certain Patent Rights and Technical Information (as defined below) invented or developed in the course of certain research conducted under the direction of Stephen D. Wilson, Sue Fletcher, Graham McClorey, Abbie Adams and Penny Meloni (hereinafter collectively referred to as the “Inventors”).

B. Certain of the Patent Rights and Technical Information had been previously assigned by UWA to SmithKline Beecham Corporation doing business as GlaxoSmithKline (“GSK”), but have, as of the Effective Date, been reassigned by GSK to UWA.

C. Licensee is in the process of developing various products for the treatment of muscular dystrophy arising from defects in the dystrophin gene by inducing the skipping of certain exons in such gene for which the Patent Rights and Technical Information may be useful.

D. UWA and Licensee entered into a certain Exclusive License Agreement, dated as of the Effective Date (the “Prior License Agreement”), pursuant to which UWA granted to Licensee certain exclusive license rights under certain patent rights and technical information relating to the treatment of Duchenne muscular dystrophy by inducing the skipping of certain specified exons or blocks of exons through the use of certain specified antisense sequences (the “Prior License Rights”).

E. Licensee and UWA desire to expand the Prior License Rights to allow Licensee to conduct research in the Field of Use, and to develop, manufacture, use and sell Products in the Field of Use, using the Patent Rights and Technical Information (as each term is defined below) in accordance with the terms of this Agreement, and UWA desires to have the Patent Rights and the Technical Information developed, used and commercialized in the Field of Use by Licensee. Other than the rights expressly granted by UWA hereunder within the Field of Use, Licensee acknowledges that UWA shall retain all other rights with respect to the Patent Rights and the Technical Information.

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

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

1. DEFINITIONS

1.1 “Affiliate” or “Affiliates” shall mean any corporation, person or entity, which controls, is controlled by, or is under common control with, a party to this Agreement without regard to stock or other equity ownership. For purposes hereof, the terms “control” and “controls” mean the possession, direct or indirect, of the power to direct or cause the direction of the management and policies of a corporation, person or entity, whether through the ownership of voting securities, by contract or otherwise.

1.2 “Confidential Information” shall mean any confidential or proprietary information furnished by one party (the “Disclosing Party”) to the other party (the “Receiving Party”) in connection with this Agreement or, prior to the Restatement Date, pursuant to the Prior License Agreement or the Mutual Confidentiality Agreement dated October 1, 2008 between the parties, including, without limitation, all specifications, know-how, trade secrets, technical information, drawings, software, models, business information and patent applications pertaining to the Patent Rights and Technical Information, and as further provided in Section 10 hereof.

1.3 “EMA” shall mean the European Medicines Agency, or any successor agency thereof.

1.4 “FDA” shall mean the United States Food and Drug Administration, or any successor agency thereof.

1.5 “Field of Use” shall mean the treatment of muscular dystrophies arising from defects in the dystrophin gene or in the transcription or translation thereof, including without limitation Duchenne and Becker muscular dystrophies.

1.6 “Improvements” shall mean all unpatented, patentable and patented inventions, discoveries, designs, apparatuses, systems, machines, methods, processes, uses, devices, models, composition of matter, technical information, trade secrets, know-how, codes, programs or configurations of any kind that relate to (i) the use of antisense oligonucleotides or other compounds to induce exon-skipping and that have application in the Field of Use, or (ii) to compositions or methods useful in connection with subsection (i), in each case that are conceived by or on behalf of UWA and are owned or controlled by UWA after the Restatement Date.

1.7 “Net Sales” shall mean the total invoiced sales price and/or value of other consideration received for Products sold by Licensee, its Affiliates or sublicensees, less (a) sales taxes or other taxes, (b) actual shipping and insurance costs, (c) actual rebates, credits, or refunds for returned or defective Products, (d) trade discounts and quantity discounts or retroactive price reductions, (e) rebates, credits, and chargeback payments (or the equivalent thereof) actually granted to managed health care organizations, wholesalers, or to federal, state/provincial, local and other governments, including their agencies, purchasers, and/or reimbursers, or to trade customers, and (f) any import or export duties, tariffs, or similar charges incurred with respect to the import or export of Products into or out of any country in the Territory. Products will be considered “sold” when put into use, sold, leased or otherwise transferred and a “sale” shall be deemed to have occurred upon first use, shipment, invoicing or receipt of payment, whichever

shall first occur. Notwithstanding the foregoing, Net Sales shall not include, and shall be deemed zero with respect to, (i) the actual distribution of reasonable quantities of promotional samples of Products, and (ii) Products provided for clinical trials or research purposes at cost or at no charge. Notwithstanding the foregoing, amounts invoiced by Licensee or its Affiliates, or their respective sublicensees, for the sale of Products among Licensee or its Affiliates or their respective sublicensees for resale shall not be included in the computation of Net Sales hereunder. Notwithstanding the foregoing, in the event that a Product is sold by Licensee, its Affiliates or sublicensees as part of a combination product or bundled product (“Combination Product”), the Net Sales of such Product, for the purposes of determining Royalty payments due under this Agreement, shall be determined by multiplying the Net Sales (as originally defined above) of the Combination Product by the fraction $A/(A+B)$, where A is the average sale price of the Product when sold separately in finished form in any country in which the Combination Product is sold and B is the average sale price of the other product(s) included in the Combination Product when sold separately in finished form, so that A+B is the average sale price of the Combination Product(s) together, in the country in which the Combination Product is sold, in each case during the applicable Royalty reporting period in which sales of both occurred, or, if sales of both the Product and the other product(s) did not occur in such period, then in the most recent Royalty reporting period in which sales of both occurred. In the event that such average sale price cannot be determined for both the Product and such other product(s) in the Combination Product, Net Sales for the purposes of determining Royalty payments with respect to such Combination Product shall be mutually agreed by the parties based on the relative value contributed by each component, such agreement not to be unreasonably withheld.

1.8 “Patent Rights” shall mean, subject to Section 2.4, (i) those patent applications and patents listed in Schedule 1.8, (ii) all other patent applications and patents owned or controlled by UWA as of the Restatement Date that claim inventions relating to (A) the use of antisense oligonucleotides to induce exon-skipping and that have application in the Field of Use, or (B) to compositions or methods useful in connection with subsection (A), and (iii) all patents and/or patent applications (including provisional patent applications) existing as of the Restatement Date in any country corresponding to any of the foregoing, and all national phases, divisions, continuations, continuations-in-part, reissues, reexaminations, supplementary protection certificates and extensions thereof, whether domestic or foreign, and any patent that issues thereon. The Patent Rights listed in Schedule 1.8 are all owned by UWA.

1.9 “Phase II Trial” shall mean a controlled clinical study conducted to evaluate the effectiveness of a Product for the treatment of muscular dystrophy, for example by testing muscle function or endurance, in patients having muscular dystrophy and to determine the common short-term side effects and risks.

1.10 “Phase III Trial” shall mean, relative to a Phase II Trial, expanded controlled and uncontrolled trials after preliminary evidence suggesting effectiveness of the Product for treatment of muscular dystrophy has been obtained, and intended to gather additional information to evaluate the overall benefit-risk relationship of the Product and to provide an adequate basis for applying for Regulatory Approval for commercial sales of the Product.

1.11 “Product” or “Products” shall mean any human therapeutics, diagnostics (including algorithms or any components thereof), bioinformatics and any other human health care products and/or services covered by the Patent Rights, including without limitation those products targeting the exons listed on Exhibit A.

1.12 “Regulatory Approval” shall mean all approvals (including pricing and reimbursement approvals), licenses, registrations or authorizations by any regulatory authority, necessary for the manufacture and sale of a Product in a regulatory jurisdiction in the Territory.

1.13 “Technical Information” shall mean know-how, trade secrets, unpublished patent applications, software, bioinformatics, unpatented technology, technical information, statistical information and analyses, biological materials, chemical reagents, preclinical and clinical information, and the like, in written, electronic or other form, now known or hereafter developed, whether or not patentable, in each case relating to (i) the use of antisense oligonucleotides to induce exon-skipping and that have application in the Field of Use, (ii) to compositions or methods useful in connection with subsection (i), or (iii) information disclosed in the Patent Rights, in each case that are conceived in whole or in part by or on behalf of UWA and are owned or controlled by UWA after the Restatement Date.

1.14 “Territory” shall mean the entire world.

1.15 “Valid Claim” shall mean a claim of an issued patent included within the Patent Rights, which claim has not (a) lapsed, been canceled or become abandoned, (b) been declared invalid or unenforceable by a non-appealable decision or judgment of a court or other appropriate body or authority of competent jurisdiction (other than with respect to any petition or writ of certiorari to the Supreme Court of the United States), or (c) been admitted to be invalid or unenforceable through reissue, disclaimer or otherwise.

2. LICENSE

2.1 Grant of Exclusive Rights. Subject to the terms of this Agreement, UWA hereby grants to Licensee, and Licensee hereby accepts from UWA, the exclusive, worldwide license, with the right to grant sublicenses (subject to the terms of Section 2.3 hereof), during the term of this Agreement (as provided in Section 6.1 hereof) to conduct research in the Field of Use using the Patent Rights and the Technical Information and to develop, use, make, have made, practice, import, carry out, manufacture, have manufactured, offer for sale, sell and/or have sold Products in the Field of Use in the Territory using the Patent Rights and the Technical Information; provided that UWA retains the right under the Patent Rights and Technical Information to conduct noncommercial research (with any Improvements resulting from such noncommercial research by UWA being subject to Section 2.4 and other relevant provisions of this Agreement). For clarity, UWA retains all rights under the Patent Rights and the Technical Information outside of the Field of Use.

2.2 Diligence.

(a) Subject to the terms of Section 4.5, Licensee shall use commercially reasonable efforts in pursuing the development, commercialization and marketing of Products. Without limiting the foregoing, Licensee shall be deemed to have exercised commercially reasonable efforts, the diligence requirements of this Section 2.2(a) shall be deemed to have been

met, and UWA may not issue a Diligence Breach Notice pursuant to Section 2.2(b), if, on a Product-by-Product basis, Licensee, together with its Affiliates and sublicensees:

(1) is actively engaged in, or has plans to engage in, pre-clinical or clinical development efforts with respect to, or commercialization of, such Product in any country or jurisdiction within the Territory;

(2) as to the Product known as AVI-4658, meets the respective requirements set forth on Schedule 2.2, with each such requirement being deemed a separate and independent condition (each, a “Milestone”); provided that if patent infringement issues prevent Licensee from meeting any such Milestone, then the timing for such Milestone shall be tolled until such issues are resolved and the parties shall in good faith negotiate and agree on an appropriate modification to the timing of such Milestone, if necessary;

(3) as to the Product known as AVI-4658, fails to meet any Milestone designated in Schedule 2.2 hereto, but, after the parties meet to discuss the status of Licensee’s development efforts, Licensee demonstrates that Licensee’s efforts amounted to commercially reasonable efforts under the circumstances, in which case the parties shall in good faith negotiate and agree on an appropriate modification to the relevant Milestone(s); or

(4) as to the Product known as AVI-4658, fails to meet any Milestone designated in Schedule 2.2 hereto, and the proviso in subsection (2) does not apply, but Licensee, its Affiliates or Sublicensees initiate a [†††] trial of a different Product within [†††] after the date upon which Licensee, its Affiliate or Sublicensee failed to meet such Milestone, in which case the Milestones obligations designated in Schedule 2.2 hereto shall apply with respect to such different Product instead of AVI-4658 for the purposes of this Section 2.2; provided that if patent infringement issues prevent Licensee from meeting any such Milestone for such different Product, then the timing for such Milestone shall be tolled until such patent infringement issues are resolved or the parties in good faith negotiate and agree on an appropriate modification to the timing of such Milestone.

(b) Subject to the terms of Section 2.2(a), if UWA believes that Licensee has failed to meet its diligence obligations as set forth in Section 2.2(a), UWA may give Licensee written notice of the deficiency (“Diligence Breach Notice”). Licensee shall thereafter have one hundred and twenty (120) days to cure the deficiency. If Licensee fails to cure the deficiency within such one hundred and twenty (120) day period, UWA may terminate this Agreement with respect to the relevant Product upon written notice to Licensee, *provided, however*, that UWA may not terminate this Agreement with respect to any Product as to which Licensee is actively engaged in, or has plans to engage in, pre-clinical or clinical development efforts or commercialization in any country or jurisdiction within the Territory. Such right to terminate this Agreement as to a given Product shall be UWA’s sole remedy for Licensee’s breach of this Section 2.2.

2.3 Right to Sublicense or Assign Rights. Licensee shall have the right to grant sublicenses consistent with this Agreement. Licensee shall keep UWA reasonably informed with respect to the progress of any relations entered into with any sublicensees. As an express condition of any such sublicense, any such sublicensee shall be required to agree in writing to be

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

bound by commercially reasonable royalty reporting and recordkeeping, indemnification and inspection provisions, and the applicable provisions of this Agreement, including, without limitation, those pertaining to the use of UWA's name and marks, indemnification of UWA and the use of UWA's Confidential Information. Licensee will be responsible for enforcing each sublicensee's obligations under its sublicense. Licensee understands and agrees that none of its sublicenses hereunder shall reduce in any manner any of its obligations set forth in this Agreement.

2.4 Future Rights. UWA shall notify Licensee of any Improvements promptly after the identification or disclosure thereof. All patent applications and patents claiming such Improvements shall automatically be included in the Patent Rights and licensed to Licensee pursuant to Section 2.1.

2.5 Commercialization in Australia. After Licensee has received Regulatory Approval in Australia with regard to a Product, Licensee shall use commercially reasonable efforts to commercialize such Product in Australia during the term of this Agreement, *provided, however*, that Licensee shall have no obligation to commercialize, or continue to commercialize, any Product in Australia under this Section 2.5 if (i) Licensee has a good faith belief that such commercialization would infringe or misappropriate any third party's intellectual property rights, or (ii) such Product does not qualify for reimbursement in Australia at a rate equal to or greater than the applicable rate established in the European Union for (1) such Product, or (2) a pharmaceutical product mutually agreed by the parties to be substantially similar to such Product with respect to reimbursement, if no reimbursement rate has been established in the European Union for such Product. If subsection (ii) applies, UWA may identify and propose to Licensee third parties who, notwithstanding such reimbursement situation, may desire to obtain a sublicense to develop and/or commercialize such Product in Australia. If UWA proposes any such potential sublicensees to Licensee, Licensee shall discuss in good faith the terms of such a sublicense with such third party for up to sixty (60) days.

3. REPRESENTATIONS AND WARRANTIES

3.1 UWA. UWA represents and warrants to Licensee that:

(a) UWA (i) is a body corporate established pursuant to the provisions of The University of Western Australia Act 1911, duly organized, validly existing and in good standing under the laws of Australia, (ii) has the corporate power and authority to enter into this Agreement and to perform its obligations hereunder, and (iii) has taken sufficient steps such that the execution and delivery of this Agreement by UWA and the performance by UWA of its obligations hereunder have been duly authorized by all necessary corporate action;

(b) to the best of UWA's knowledge, at the Restatement Date, there are no claims, judgments or settlements to be paid by UWA with respect to the Patent Rights or Technical Information or pending claims or litigation relating to the Patent Rights or Technical Information;

(c) with respect to the Patent Rights, UWA has been assigned all right, title and interest from the Inventors and UWA is listed as the sole owner of record in the records of the United States Patent and Trademark Office and any foreign patent offices with respect to Patent Rights that consist of applications or registrations with such offices,

(d) UWA has the right to grant the rights granted to Licensee hereunder and to perform UWA's obligations hereunder, in each case without the consent or approval of any third party;

(e) UWA has not granted, and will not grant, licenses to the Patent Rights, Technical Information or Improvements to any third party that would conflict with or otherwise compromise the rights granted to Licensee hereunder;

(f) the Patent Rights have been duly prepared, filed, prosecuted, obtained, and maintained in accordance with all applicable laws, rules, and regulations;

(g) to the best of UWA's knowledge, and except as specified on Schedule 3.1, no third party's intellectual property rights would be infringed or misappropriated by the practice of the Patent Rights in general and no third party is infringing or misappropriating the Patent Rights;

(h) UWA does not own or control any patents or patent applications other than the Patent Rights that currently, or when issued, would be infringed by the making, using, offering for sale, selling, or importing of any product or process covered by a claim within the Patent Rights;

(i) this Agreement constitutes the legal, valid and binding obligation of UWA, enforceable against UWA in accordance with its terms, subject only to (i) applicable bankruptcy, insolvency, reorganization, moratorium or similar laws affecting the enforceability of creditors' rights generally, (ii) the limitation that the remedy of specific performance or injunctive relief is subject to the discretion of the court or arbitrator before which any proceeding therefor may be brought, and (iii) general legal and equitable principles of good faith, fair dealing and equity; and

(j) to the best of UWA's knowledge, neither the execution or delivery of this Agreement by UWA, nor the performance by UWA of its obligations hereunder, (i) requires the consent or approval of any third party; (ii) shall constitute a default under any material contract by which UWA or any of its material assets is bound (or any event which, with notice or lapse of time, or both, would constitute such a default); or (iii) shall constitute a violation of any judgment, order or decree of any court, arbitrator, governmental agency or authority binding upon UWA.

For the avoidance of doubt:

- UWA does not warrant or represent that the Patent Rights, Technical Information or Improvements or any part thereof are or will be valid under this Agreement.
- UWA makes no warranties or representations regarding any scientific information, including without limitation as to the accuracy or completeness thereof, provided in respect of this Agreement.

-
- UWA does not warrant the applicability, utility or usability of the Patent Rights, the Technical Information or Improvements in respect of the Products and disclaims any and all liability in respect of the application of the Patent Rights, the Technical Information or Improvements.

3.2 Licensee. Licensee represents and warrants to UWA that:

(a) Sarepta is a corporation duly organized, validly existing and in good standing under the laws of the State of Oregon, and Sarepta Netherlands is a corporation duly organized, validly existing and in good standing under the laws of the Netherlands, and each of Sarepta and Sarepta Netherlands has the corporate power and authority to enter into this Agreement and to perform its obligations hereunder;

(b) the execution and delivery of this Agreement by Licensee and the performance by Licensee of its obligations hereunder have been duly authorized by all necessary corporate action;

(c) this Agreement constitutes the legal, valid and binding obligation of Licensee, enforceable against Licensee in accordance with its terms, subject only to (i) applicable bankruptcy, insolvency, reorganization, moratorium or similar laws affecting the enforceability of creditors' rights generally, (ii) the limitation that the remedy of specific performance or injunctive relief is subject to the discretion of the court or arbitrator before which any proceeding therefor may be brought, and (iii) general legal and equitable principles of good faith, fair dealing and equity; and

(d) neither the execution or delivery of this Agreement by Licensee, nor the performance by Licensee of its obligations hereunder, (i) requires the consent or approval of any third party; (ii) shall constitute a default under any material contract by which Licensee or any of its material assets is bound (or any event which, with notice or lapse of time, or both, would constitute such a default); or (iii) shall constitute a violation of any judgment, order or decree of any court, arbitrator, governmental agency or authority binding upon Licensee.

4. CONSIDERATION

In consideration of the execution and delivery by UWA of this Agreement and the rights and licenses granted to Licensee hereunder, Licensee agrees as follows:

4.1 License Fees.

(a) Within thirty (30) days of the Restatement Date, Licensee shall pay to UWA a one-time, upfront license fee in an amount equal to [\$\$\$] U.S. Dollars (USD [\$\$\$]);

(b) Within thirty (30) days of the Restatement Date, Licensee shall pay to UWA a one-time fee for patent filing costs in an amount equal to [\$\$\$] U.S. Dollars (USD [\$\$\$]); and

(c) Within ninety (90) days of each of the second, third and fourth anniversaries of the Restatement Date, Licensee shall pay to UWA a license maintenance fee in an amount equal to [\$\$\$] U.S. Dollars (USD [\$\$\$]).

[\$\$\$] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

4.2 Payment of Royalties.

(a) Licensee shall pay to UWA on a Product-by-Product and a country-by-country basis, royalty fees (each, a “Royalty” and collectively, the “Royalties”) equal to [†††] % of aggregate Net Sales of all Products by Licensee, its Affiliates or sublicensees.

(b) Royalties shall accrue and be payable by Licensee on a quarterly basis within forty-five (45) days following the end of each calendar quarter in which any Products generating Net Sales were sold. Each payment of Royalties shall be accompanied by a statement setting forth in reasonable detail the number and each type of Product sold and the Net Sales applicable thereto in the applicable calendar quarter. The Products shall be considered as being sold for the purpose of the calculation of Royalties under this Agreement when the payments for such Products have been received by Licensee. Except as otherwise provided in Section 4.6, all Royalties shall be paid in United States Dollars.

(c) Licensee shall create and maintain complete and accurate records and documentation concerning all Net Sales of Products in sufficient detail to enable the Royalties payable hereunder to be determined. Licensee shall retain such records and documentation for not less than three (3) years from the date of their creation. During the term of this Agreement and for a period of one (1) year thereafter, UWA and its representatives shall have the right to audit such records and documentation as shall pertain to the determination and payment of Royalties no more than once in any calendar year. Such examiners shall have reasonable access during regular business hours to Licensee’s offices and the relevant records, files and books of account, and shall have the right to examine any other records reasonably necessary to determine the accuracy of the Royalty calculations provided by Licensee. The costs of any such audit shall be borne by UWA, unless as a result of such inspection it is determined that the amounts payable by Licensee for any period are in error by greater than five percent (5%), in which case the costs of such audit shall be borne by Licensee. UWA shall report the results of any such audit to Licensee within forty-five (45) days of completion. Thereafter, Licensee shall promptly pay to UWA the amount of any underpayment discovered in such audit, or UWA shall credit to Licensee against future Royalty payments the amount of any overpayment discovered in such audit, as the case may be. In addition, Licensee shall pay interest on any underpayment at the rate that is the lower of (i) two percent (2%) over the rate of interest announced by Bank of America in Massachusetts (or any successor in interest thereto or any commercially equivalent financial institution if no such successor exists) to be its “prime rate,” or (ii) the highest rate permitted by applicable law, in each of cases (i) and (ii) from the date such amount was underpaid to the date payment is actually received.

4.3 Royalty Purchase.

(a) Licensee may, at its sole option, choose to terminate its obligation to pay Royalties to UWA under Section 4.2 by providing written notice thereof to UWA at any time after the first Regulatory Approval of any Product in the Territory, but prior to April 1, [†††], (the “Royalty Purchase Notice”) and agreeing to pay to UWA (i) a one-time payment of [†††] U.S. Dollars (USD [†††]) (the “Royalty Purchase Upfront Payment”), (ii) a one-time payment of [†††]

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

U.S. Dollars (USD [†††]) the first time that aggregate Net Sales of all Products in the Territory exceed [†††] U.S. Dollars (USD [†††]) in any calendar year prior to January 1, [†††], and (iii) a one-time payment of [†††] U.S. Dollars (USD [†††]) the first time aggregate Net Sales of all Products in the Territory exceed [†††] U.S. Dollars (USD [†††]) in any calendar year prior to January 1, [†††] (ii) and (iii) each, a “Royalty Purchase Milestone Payment,” and collectively with the Royalty Purchase Upfront Payment, the “Royalty Purchase Payment”), pursuant to the terms of this Section 4.3 (the “Royalty Purchase”). For clarity, if the Royalty Purchase becomes effective, if aggregate Net Sales of all Products in the Territory exceed [†††] U.S. Dollars (USD [†††]) for the first time in the same year that aggregate Net Sales of all Products in the Territory exceed [†††] U.S. Dollars (USD [†††]) for the first time, then both Royalty Purchase Milestone Payments shall be triggered and Licensee shall pay to UWA [†††] U.S. Dollars (USD [†††]). For clarity, in no event shall Licensee be obligated to pay to UWA pursuant to this Section 4.3 more than a total of [†††] U.S. Dollars (USD [†††]), if Licensee provides a Royalty Purchase Notice and each of the milestones set forth in subsections (ii) and (iii) are achieved.

(b) During the period beginning on the date upon which UWA receives the Royalty Purchase Notice and ending [†††] thereafter, unless such period is earlier terminated by written notice from UWA to Licensee (the “Royalty Purchase Period”), UWA may solicit offers to assign to a third party [†††] its right, [†††] to receive Royalties from Licensee under Section 4.2 (the “Royalty Rights”). If, during the Royalty Purchase Period, [†††] offer[†††] to UWA compensation in exchange for the Royalty Rights [†††] (each, a “Competing Bid”), UWA shall notify Licensee, in writing, of the [†††] Competing Bid received during the Royalty Purchase Period (the “Leading Bid”) [†††] after receiving such Leading Bid. During the [†††] period beginning upon Licensee’s receipt of such notification of a Leading Bid (the “Decision Period”), Licensee may provide written notice to UWA that it agrees to the Royalty Purchase on the terms of the Leading Bid instead of the Royalty Purchase Payment under Section 4.3(a) (“Match Notice”). If Licensee does not provide a Match Notice to UWA during the Decision Period, UWA may assign the Royalty Rights to the third party that made the Leading Bid on the terms of the Leading Bid, in which case Licensee shall have no further right to exercise the Royalty Purchase under Section 4.3(a) [†††]. If no Leading Bid is received during the Royalty Purchase Period, UWA may not assign the Royalty Rights to any third party. Licensee may not exercise the Royalty Purchase and shall have no obligation to pay to UWA any portion of the Royalty Purchase Payment during the Royalty Purchase Period.

(c) [†††], if UWA has not exercised its right to assign the Royalty Rights to a third party under Section 4.3(b), Licensee may exercise the Royalty Purchase at any time by providing written notice thereof to UWA and (i) by making any payments necessary pursuant to the terms of the Leading Bid specified in the latest Match Notice, or (ii) if no Match Notice was provided by Licensee to UWA, by paying the Royalty Purchase Upfront Payment within [†††] days after UWA’s receipt of Licensee’s notice exercising the Royalty Purchase, and each Royalty Purchase Milestone Payment within [†††] days after December 31 of the year for which such Royalty Purchase Milestone Payment was earned.

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

(d) If the Royalty Purchase becomes effective, Licensee shall have no further obligation under Section 4.2 to pay to UWA any Royalty on sales of any Product that occur after the date on which UWA received the Royalty Purchase Notice. To the extent any Royalties have been paid by Licensee to UWA pursuant to Section 4.2 for sales of Product after UWA's receipt of the Royalty Purchase Notice, such Royalties shall be fully credited toward any payment made by Licensee to UWA pursuant to Section 4.3(c). Notwithstanding anything in this Section 4.3 to the contrary, Licensee's obligations under Section 4.4 shall remain in effect even in the event the Royalty Purchase becomes effective.

4.4 Milestone Fees.

(a) Licensee shall pay to UWA the following fees (each, a "Milestone Fee"), which shall be determined and paid within ninety (90) days after the occurrence of each of the following corresponding events (each, a "Milestone Event"):

(i) With respect to the Product known as of the Restatement Date as eteplirsen ("Eteplirsen"), [†††] U.S. Dollars (USD [†††]) upon first commercial sale in any country or jurisdiction within the Territory.

(ii) With respect to Products other than Eteplirsen, each of the following Milestone Fees shall be paid, if ever, for each such Product that achieves the corresponding Milestone Event; *provided, however*, that no such Milestone Fee shall be paid by Licensee more than [†††] times, regardless of the number of Products that achieve the corresponding Milestone Event:

(1) [†††] U.S. Dollars (USD [†††]) upon initiation of a Phase II Trial of a Product;

(2) [†††] U.S. Dollars (USD [†††]) upon initiation of a Phase III Trial of a Product;

(3) [†††] U.S. Dollars (USD [†††]) upon Regulatory Approval of a Product by the first of the FDA or the EMA.

(b) If any Milestone Event is achieved with respect to a given Product, all previously listed Milestone Events, if not already achieved for such Product, shall be considered to be simultaneously achieved and Licensee shall pay to UWA the aggregate Milestone Fees associated with all such Milestone Events. As an example, and solely for clarity, if no Phase II Trial is required or initiated for a particular Product, Licensee shall pay to UWA an aggregate of [†††] U.S. Dollars (USD [†††]) within ninety (90) days after initiation of a Phase III Trial for such Product. Additionally, for clarity, in no event shall Licensee be obligated to pay to UWA more than [†††] U.S. Dollars (USD [†††]) pursuant to this Section 4.4.

(c) Notwithstanding anything to the contrary in this Section 4.4, if a Valid Claim specifically covering a Product has not issued in the United States or European Union at

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

the time a Milestone Event for such Product is achieved, Licensee shall only be obligated to pay to UWA [†††] percent ([†††]%) of the corresponding Milestone Fee until such time, if any, that such Valid Claim is granted in the United States or European Union.

4.5 Infringement. In the event that Licensee is prevented from developing, manufacturing or commercializing one or more Products in a particular jurisdiction in the Territory as a result of patent infringement issues, all of Licensee's obligations with respect to such Products in such jurisdiction, including, without limitation, Royalty and other obligations, shall be suspended unless and until such patent infringement issues are resolved. In the event that any such issues are not resolved during the term of the Agreement, or in the event that such issues are resolved in a manner that would continue to prevent Licensee from developing, manufacturing or commercializing such Products, then Licensee shall have no further obligations hereunder with respect to such Products.

4.6 Currency Transfer Restrictions. If any restrictions on the transfer of currency exist in any country or other jurisdiction so as to prevent Licensee from making payments to UWA, Licensee shall take all commercially reasonable steps to obtain a waiver of such restrictions or to otherwise enable Licensee to make such payments. If Licensee is unable to do so, Licensee shall make such payments to UWA in a bank account or other depository designated by UWA in such country or jurisdiction, which payments shall be in the local currency of such country or jurisdiction, unless payment in United States Dollars is permitted. Any payment by Licensee to UWA in currencies other than United States Dollars shall be calculated using the appropriate foreign exchange rate for such currency quoted in the *Wall Street Journal* for the close of business of the last banking day prior to the date on which such payment is being made.

4.7 Fair Market Value. UWA acknowledges and agrees that the Royalties, Milestone Fees and other obligations of Licensee under this Agreement constitute fair market value for the rights granted to Licensee under this Agreement based on arms'-length negotiations with Licensee.

4.8 Withholding Tax. Licensee shall make all payments to UWA under this Agreement without deduction or withholding for taxes except to the extent that any such deduction or withholding is required by applicable law in effect at the time of payment. Any tax required to be withheld on amounts payable under this Agreement shall promptly be paid by Licensee on behalf of UWA to the appropriate governmental authority, and Licensee shall furnish UWA with proof of payment of such tax. Any such tax required to be withheld will be borne by Licensee. Each party will cooperate with respect to all documentation required by any taxing authority or reasonably requested by Licensee to secure a reduction in the rate of applicable withholding taxes.

5. PATENT RIGHTS

5.1 Prosecution of Existing Patent Rights. Licensee shall assume full responsibility for the application, maintenance, reexamination, reissue, opposition and prosecution of any kind (collectively "Prosecution") relating to the Patent Rights in the Territory, including without limitation those Patent Rights claiming Improvements, including, but not limited to, payment of all costs, fees and expenses related thereto. Licensee shall provide UWA with copies of any and

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

all material or communications with the United States Patent and Trademark Office or any foreign patent office. In the event that Licensee elects to abandon the Prosecution or maintenance of any patent or patent application included in such Patent Rights, Licensee shall notify UWA of such election at least thirty (30) days before a final due date which would result in abandonment or bar of patentability of the patent or patent application and, in such event, UWA may, at its sole option and expense, continue Prosecution or maintenance of the Patent Rights.

5.2 Expenses. Licensee shall pay all expenses resulting from its obligations in Section 5.1 hereof. UWA shall exercise reasonable efforts to cause the Inventors to cooperate fully with Licensee with respect to the Prosecution, maintenance and protection of the Patent Rights, including without limitation any patents or patent applications covering the Improvements.

6. TERM AND TERMINATION

6.1 Term. Unless earlier terminated as provided in Section 6.2 hereof, the term of this Agreement shall commence on the Effective Date and shall expire, on a country-by-country basis, on the date upon which the last to expire Valid Claim of the Patent Rights shall expire in such country.

6.2 Termination. Except as provided by Section 6.3 hereof, this Agreement shall terminate, in its entirety or on a Product-by-Product basis (as provided in this Section 6.2) upon the earliest to occur of the following:

(a) Subject to the terms of Section 2.2 and Section 4.5, upon sixty (60) days' written notice from UWA if, within such sixty (60) day period, Licensee shall fail to cure fully any breach or default of any material obligation under this Agreement with respect to a given Product or Products, as described in such written notice detailing the facts of such breach with reasonable specificity; *provided, however*, that Licensee may avoid such termination if, before the end of such sixty (60) day period, such breach or default has been cured by Licensee to the reasonable satisfaction of UWA;

(b) Upon sixty (60) days' written notice from Licensee if, within such sixty (60) day period, UWA shall fail to cure fully any breach or default of any material obligation under this Agreement as described in such written notice detailing the facts of such breach with reasonable specificity; *provided, however*, that UWA may avoid such termination if, before the end of such sixty (60) day period, such breach or default has been cured by UWA to the reasonable satisfaction of Licensee;

(c) Upon the mutual written agreement of the parties hereto (such termination to be effective as of the date mutually agreed upon in such written agreement);

(d) Immediately upon Licensee passing a resolution for winding-up (otherwise than for the purposes of a solvent amalgamation or reconstruction where the resulting entity is at least as credit-worthy as the Licensee and assumes all of the obligations of the Licensee under this Agreement) or a court shall make an order to that effect; or if a liquidator, receiver, administrator, administrative receiver, manager, trustee, or similar officer is appointed over any of the assets of the Licensee; or

(e) Immediately by Licensee, in its sole discretion, upon notice to UWA that Licensee is no longer desirous of commercializing a given Product or Products. For clarity, if Licensee terminates this Agreement pursuant to this Section 6.2(e) as to a given Product, Licensee's license under Section 2.1 and its other rights under this Agreement shall terminate solely with respect to such Product, but Licensee's license under Section 2.1 and its other rights under this Agreement shall continue in full force and effect thereafter with respect to all other Products.

6.3 Obligations Upon Termination. Upon any termination of this Agreement pursuant to Sections 2.2 or 6.2 hereof, nothing herein shall be construed to release any party from any liability for any obligation incurred through the effective date of termination or for any breach of this Agreement prior to the effective date of such termination. Licensee may, for a period of one (1) year after the effective date of such termination, sell all tangible Products customarily classified as "inventory" that it has on hand at the date of termination, subject to payment by Licensee to UWA of the applicable Royalty under Sections 4.2 and 4.3 hereof.

6.4 Effect of Termination. In the event of any termination of this Agreement pursuant to Sections 2.2 or 6.2 hereof, where such termination has not been caused by any action or inaction on the part of any sublicensee of Licensee or by any breach by such sublicensee of its obligations under its sublicense from Licensee, such termination of this Agreement shall be without prejudice to the rights of each non-breaching sublicensee of Licensee and each non-breaching sublicensee shall be deemed to be a licensee of UWA thereunder, and UWA shall be entitled to all rights, but shall not be subject to any obligations (other than the grant of license and appurtenant obligations under this Agreement to the extent provided for in such sublicense) of Licensee thereunder.

6.5 Right to Institute Legal Actions. Notwithstanding the provisions of Section 6.2 hereof, UWA, on the one hand, and Licensee, on the other hand, may institute any other legal action or pursue any other remedy against the other party permitted by applicable law if the other party does not substantially cure any breach or default of any material obligation as provided herein.

6.6 Reversion of Rights. Notwithstanding anything to the contrary set forth herein (including, but not limited to, Section 5 hereof), full responsibility for Prosecution of the Patent Rights shall, at the option of UWA and at its sole expense from the date of reversion, revert to UWA upon any termination of this Agreement. Upon any termination of this Agreement, Licensee shall have no further obligation to Prosecute the Patent Rights under Section 5.1 and shall not be responsible for any expenses relating to Prosecution of Patent Rights incurred after the effective date of such termination.

7. INFRINGEMENT AND PROSECUTION BY THIRD PARTIES

7.1 Enforcement. Licensee shall have the first right, but not the obligation, to enforce, at its sole expense, any Patent Rights to the extent licensed hereunder against

infringement by third parties and shall notify UWA in writing in advance of all such enforcement efforts. Upon Licensee's undertaking to pay all expenditures reasonably incurred by UWA, UWA shall reasonably cooperate in any such enforcement and, as necessary, join as a party therein. Licensee shall reimburse UWA for all expenses, including reasonable attorneys' fees, incurred in connection with any such enforcement. In the event that Licensee does not file suit against or commence and conclude settlement negotiations with a substantial infringer of Patent Rights within ninety (90) days of receipt of a written demand from UWA that Licensee bring suit, then the parties will consult with one another in an effort to determine whether a reasonably prudent licensee would institute litigation to enforce the patent in question in light of all relevant business and economic factors (including, but not limited to, the projected cost of such litigation, the likelihood of success on the merits, the probable amount of any damage award, the prospects for satisfaction of any judgment against the alleged infringer, the possibility of counterclaims against the parties hereto, the impact of any possible adverse outcome on Licensee and the effect any publicity might have on the parties' respective reputations and goodwill). If, after such process, it is determined that a suit should be filed and Licensee does not file suit or commence settlement negotiations forthwith against the infringer, then UWA shall have the right, at its own expense, to enforce any Patent Rights licensed hereunder on behalf of itself and Licensee. Any amount recovered in any such action or suit, whether by judgment or settlement, shall be paid to or retained entirely by whichever party brought the action, or where both parties participate in such action or suit, all such amounts shall be allocated to each party in the ratio of expenses incurred, after first paying each party's out-of-pocket expenses, including reasonable attorneys' fees.

7.2 Defense of Patent Rights. In the event that any Patent Rights are the subject of a legal action seeking declaratory relief or of any reexamination or opposition proceeding instituted by a third party, then Licensee shall have the first right to conduct such defense, bear the expenses, including attorneys' fees, associated with such defense and shall receive, in its entirety, any recoupment of expenses. UWA shall assist and cooperate with Licensee in such proceedings and shall exercise reasonable efforts to cause the Inventors to assist and cooperate fully. During the term of this Agreement, UWA shall maintain and make available to Licensee laboratory notebooks relating to the inventions claimed in the Patent Rights.

7.3 Third Party Patent Rights. If Licensee reasonably determines that any Product infringes upon the rights of a third party because of the use of the Patent Rights, Technical Information or Improvements in the manufacture, use or sale of such Product, and, as a result, Licensee elects to oppose, seek reexamination of, pursue declaratory relief with respect to and/or undertake other legal action with respect to such third party's patent(s) or patent application(s) before a patent office and/or the courts of any jurisdiction in the Territory (collectively "Opposition"), then UWA shall assist and cooperate with Licensee in any such Opposition. UWA shall exercise reasonable efforts to cause the Inventors to cooperate fully with Licensee at Licensee's expense with respect to any Opposition.

8. INDEMNIFICATION

8.1 Indemnification by Licensee. UWA shall not be liable for any loss or damage sustained by Licensee or any other person directly or indirectly from or in connection with Licensee's use, licence or commercialisation of any part of the Products, Patent Rights,

Improvements or Technical Information, except to the extent that such loss or damages results from the negligence or willful acts or omissions of UWA. Subject to Section 8.2 hereof, Licensee hereby releases and indemnifies UWA, its officers, employees and agents from and against all actions, claims, proceedings and demands whatsoever, including through contract and tort which may be made or brought by any person, body or authority against it or them or any of them in respect of any loss, injury or damage including death and consequential loss ("Losses") arising out of Licensees' use of the Products, Patent Rights, Improvements or Technical Information, except to the extent that such Losses result from the negligence, or willful acts or omissions of UWA.

8.2 Indemnification by UWA. Subject to Section 8.3 hereof, UWA shall hold harmless, defend and indemnify Licensee and each of its officers, directors, employees and agents from and against any and all claims, damages, losses, liabilities, costs and expenses (including reasonable attorneys' fees and expenses and costs of investigation, whether or not suit is filed) suffered or incurred in connection with any negligence, willful acts or omissions or breach on the part of UWA directly resulting from the assignment or reassignment of the Patent Rights between UWA and GSK.

8.3 Notice of Claim. UWA shall promptly notify Licensee in writing of any claim, action or material threat thereof brought against UWA in respect of which indemnification may be sought hereunder, and, to the extent allowed by law, shall reasonably cooperate with Licensee in defending or settling any such claim or action. No settlement of any claim, action or threat thereof received by UWA and for which UWA intends to seek indemnification (for itself or on behalf of any other party) shall be made without the prior joint written approval of UWA and Licensee.

9. USE OF NAMES

Neither party shall, unless as required by any law or governmental regulation, use the name of the other party and/or any of its trademarks, service marks, trade names or fictitious business names without express prior written consent of the other party.

10. CONFIDENTIALITY

10.1 Non-Disclosure. The parties hereto shall keep the terms of this Agreement and all business and scientific discussions relating to the business of the parties strictly confidential. It may, from time to time, be necessary for the parties, in connection with performance under this Agreement, to disclose Confidential Information (including know-how) to each other. The Receiving Party (as defined in Section 1.2 hereof) shall keep in strictest confidence the Confidential Information of the Disclosing Party (as defined in Section 1.2 hereof), using the standard of care it normally uses for information of like character, but in no case less than a reasonable standard of care, and shall not disclose the Confidential Information to any third party or use it except as expressly authorized by the prior written consent of the Disclosing Party or as otherwise permitted by this Agreement; *provided, however*, that Licensee may disclose the Confidential Information received from UWA to its Affiliates and sublicensees as shall be reasonably necessary to carry out the intent of this Agreement or any sublicense granted by Licensee as contemplated by this Agreement if, but only if, such Affiliates and/or sublicensees

each execute a confidentiality agreement containing confidentiality provisions no less restrictive than those confidentiality provisions contained in this Section 10. The Receiving Party's obligation hereunder shall not apply to Confidential Information that the Receiving Party can show:

(a) Is or later becomes part of the public domain through no fault or neglect of the Receiving Party;

(b) Is received in good faith from a third party having no obligations of confidentiality to the Disclosing Party, *provided, however*, that the Receiving Party complies with any restrictions imposed by the third party;

(c) Is independently developed by the Receiving Party without use of the Disclosing Party's Confidential Information; or

(d) Is required to be disclosed by law or regulation (including, without limitation, in connection with FDA filings, SEC filings or filings with another government agency) or by the rules of a securities exchange, *provided, however*, that the Receiving Party uses reasonable efforts to restrict disclosure and to obtain confidential treatment.

10.2 Limits on Permitted Disclosures. Each party agrees that any disclosure or distribution of the other party's Confidential Information within its own organization shall be made only as is reasonably necessary to carry out the intent of this Agreement. The parties further agree that all of their respective officers, employees, agents, representatives or sublicensees to whom any Confidential Information is disclosed or distributed shall be subject to written confidentiality and non-use obligations no less restrictive than the confidentiality and non-use obligations provided for in this Section 10.

10.3 Legally Required Disclosures. If a subpoena or other legal process concerning Confidential Information is served upon any party hereto pertaining to the subject matter hereof, the party served shall notify the other party immediately, the other party shall cooperate with the party served, at the other party's expense, in any effort to contest the validity of such subpoena or other legal process. This Section 10.3 shall not be construed in any way to limit any party's ability to satisfy any disclosure of its relationship with the other party required by any governmental authority or rules of a securities exchange.

10.4 Return of Confidential Information. In the event of any termination of this Agreement, the Receiving Party shall, upon the Disclosing Party's request, promptly return all Confidential Information and any copies made thereof previously made available to the Receiving Party by the Disclosing Party, *provided, however*, that counsel of each party may retain one (1) copy of such Confidential Information to ensure compliance with this Section 10.

10.5 Remedies. Both parties acknowledge and agree that it would be difficult to measure damages for breach by either party of the covenants set forth in this Section 10, and that injury from any such breach would be incalculable, and that money damages would therefore be an inadequate remedy for any such breach. Accordingly, each party shall be entitled, in addition to all other remedies available hereunder or under law or equity, to injunctive or such other equitable relief as a court may deem appropriate to restrain or remedy any breach of such covenants.

10.6 Confidentiality Period. The obligations of confidentiality and non-use set forth in this Section 10 shall apply during the term of this Agreement and for a period of five (5) years thereafter. Notwithstanding the foregoing, any Confidential Information that is expressly labeled or noted by Licensee as a trade secret shall not be subject to such five (5) year term, but shall continue to be subject to the obligations of confidentiality and non-use set forth in this Section 10 for as long as such trade secret remains confidential.

10.7 Publicity. Notwithstanding anything to the contrary set forth herein, UWA agrees that Licensee shall have the right to issue one or more press releases announcing the execution of this Agreement and the terms hereof, the contents of which press releases shall be in Licensee's sole discretion; provided that where practicable, Licensee shall provide to UWA a reasonable opportunity to review such press releases to allow UWA to confirm their factual accuracy. UWA shall not disclose the terms of this Agreement without the prior written consent of Licensee, *provided, however*, that UWA shall not require Licensee's consent to disclose subsequently any terms of this Agreement that have already been disclosed in accordance with this Section 10.7. Furthermore, UWA shall have the right to issue a press release in Australia stating that UWA has granted a license to Licensee, which press release shall be subject to Licensee's prior written consent. Neither party may reference the other party in any other public announcements or press releases without the prior written approval of the other party, except such as may be required by law.

11. MISCELLANEOUS

11.1 Notices. Any notice, request, instruction or other document required by this Agreement shall be in writing and shall be deemed to have been given (a) if mailed with the United States Postal Service by prepaid, first class, certified mail, return receipt requested, at the time of receipt by the intended recipient, (b) if sent by Federal Express®, Airborne®, or other overnight carrier, signature of delivery required, at the time of receipt by the intended recipient, (c) if sent by electronic mail and no delivery failure notification has been received, one (1) business day after sending, or (d) if sent by facsimile transmission, when so sent and when receipt has been acknowledged by appropriate telephone or facsimile receipt, addressed as follows:

In the case of UWA to:

The University of Western Australia
35 Stirling Highway
Crawley, WA 6009
Attention: Director, Office of Industry and Innovation
Fax: +61 8 6488 2333

or in the case of Licensee to:

Sarepta Therapeutics
245 First Street Suite 1800 Cambridge,
MA 02142 USA
Attention: Chris Garabedian, Chief Executive Officer
Fax:

with a copy to:

Latham & Watkins
140 Scott Drive
Menlo Park, CA 94025
Attention: Judith Hasko
Facsimile: (650) 463-2600

or to such other address or to such other person(s) as may be given from time to time under the terms of this Section 11.1.

11.2 Governing Law. This Agreement shall be construed and enforced in accordance with the laws of the United States of America and of the State of Massachusetts, irrespective of choice of laws provisions. The parties agree that Boston, Massachusetts shall be the situs of any legal proceeding arising out of or relating to this Agreement.

11.3 Waiver. Failure of any party to enforce a right under this Agreement shall not act as a waiver of that right or the ability to assert that right relative to the particular situation involved.

11.4 Enforceability. If any provision of this Agreement shall be found by a court of competent jurisdiction to be void, invalid or unenforceable, the same shall be reformed to comply with applicable law or stricken if not so conformable, so as not to affect the validity or enforceability of the remainder of this Agreement.

11.5 Modification. No change, modification, or addition or amendment to this Agreement, or waiver of any term or condition of this Agreement, is valid or enforceable unless in writing and signed and dated by the authorized officers of the parties to this Agreement.

11.6 Entire Agreement. This Agreement constitutes the entire agreement between the parties with respect to the subject matter hereof, and replaces and supersedes as of the Restatement Date any and all prior agreements and understandings, whether oral or written, between the parties with respect to the subject matter of such agreements, including without limitation the Prior License Agreement and the Mutual Confidentiality Agreement dated October 1, 2008 between the parties.

11.7 Successors. Except as otherwise expressly provided in this Agreement, this Agreement shall be binding upon, inures to the benefit of, and is enforceable by, the parties and their respective heirs, legal representatives, successors and permitted assigns.

11.8 Construction. This Agreement has been prepared, examined, negotiated and revised by each party and their respective attorneys, and no implication shall be drawn and no provision shall be construed against any party to this Agreement by virtue of the purported identity of the drafter of this Agreement or any portion thereof.

11.9 Counterparts. This Agreement may be executed simultaneously in one or more counterparts, each of which shall constitute one and the same instrument. This Agreement may be executed by facsimile or electronic mail.

11.10 Attorneys' Fees. In the event of any action at law or in equity between the parties hereto to enforce any of the provisions hereof, the unsuccessful party to such litigation shall pay to the successful party all reasonable costs and expenses, including reasonable attorneys' fees, incurred therein by such successful party; and if such successful party shall recover a judgment in any such action or proceeding, such reasonable costs, expenses and attorneys' fees may be included in and as part of such judgment.

11.11 Assignment. This Agreement may not be assigned by either party without the prior written consent of the other party, and any such attempted assignment shall be void and of no effect, except that either party may assign this Agreement to any successor in connection with the merger, consolidation or sale of all or substantially all of its assets or that portion of its business to which this Agreement relates.

11.12 Further Assurances. At any time and from time to time after the Restatement Date, each party shall do, execute, acknowledge and deliver, and cause to be done, executed, acknowledged or delivered, all such further acts, transfers, conveyances, assignments or assurances as may be reasonably required to consummate the transactions contemplated by this Agreement.

11.13 Survival. The terms and conditions of the following provisions will survive termination or expiration of this Agreement for as long as necessary to permit their full discharge: Section 1 ("Definitions"), Section 6 ("Term and Termination") (except Sections 6.1 and 6.2), Section 8 ("Indemnification"), Section 9 ("Use of Names"), Section 10 ("Confidentiality") and Section 11 ("Miscellaneous"). The provisions set forth in Section 4 ("Consideration") also shall survive any expiration or earlier termination of this Agreement, to the extent payments thereunder are accrued but remain unpaid upon the effective date of such expiration or termination and as set forth in Section 4.2(c). Except as otherwise provided in this Section 11.13, all other provisions of this Agreement shall terminate upon the expiration or termination of this Agreement.

11.14 Joint and Several Liability. Each of Sarepta and Sarepta Netherlands shall be jointly and severally liable for Licensee's performance under this Agreement. .

[Signature page follows.]

IN WITNESS WHEREOF, the parties have caused their duly authorized representatives to execute this Agreement as of the date first above written.

“UWA”:

**THE UNIVERSITY OF WESTERN
AUSTRALIA, A BODY CORPORATE
ESTABLISHED PURSUANT TO THE PROVISIONS
OF THE UNIVERSITY OF WESTERN
AUSTRALIA ACT 1911**

By: /s/ Robyn Owens

Name: Professor Robyn Owens

Title: Deputy Vice-Chancellor (Research) The
University of Western Australia

“LICENSEE”:

SAREPTA THERAPEUTICS

By: /s/ Chris Garabedian

Name: Chris Garabedian

Title: President and CEO

SAREPTA INTERNATIONAL CV

By: /s/ Sandesh Mahatme

Name: Sandesh Mahatme

Title: President

EXHIBIT A

Exons

[†††]

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

SCHEDULE 1.8

Patent Rights

[†††]

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

SCHEDULE 2.2

Diligence Milestones

[†††]

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

SCHEDULE 3.1

Litigation

[†††]

[†††] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

SAREPTA THERAPEUTICS, INC.
EXECUTIVE EMPLOYMENT AGREEMENT

This Employment Agreement (the “**Agreement**”) is effective as of January 1, 2013 (the “**Effective Date**”) by and between Sarepta Therapeutics, Inc., formerly known as AVI BioPharma, Inc. (the “**Company**”), and Chris Garabedian (“**Executive**”). This Agreement amends and restates in its entirety that certain employment agreement by and between Executive and the Company dated as of December 17, 2010 (the “**Prior Agreement**”).

1. Duties and Scope of Employment.

(a) Positions and Duties. Executive will continue to serve as the Company’s President and Chief Executive Officer. Executive will continue to render such business and professional services in the performance of his duties as will reasonably be assigned to him by the Company’s Board of Directors (the “**Board**”).

(b) Obligations. During the Employment Term (as defined below), Executive will perform his duties faithfully and to the best of his ability and will devote his full business efforts and time to the Company. For the duration of the Employment Term, Executive agrees not to actively engage in any other employment, occupation or consulting activity for any direct or indirect remuneration without the prior approval of the Board.

2. At-Will Employment. The parties agree that Executive’s employment with the Company will continue to be “at-will” employment and may be terminated at any time with or without Cause or notice. Executive understands and agrees that neither his job performance nor promotions, commendations, bonuses or the like from the Company give rise to or in any way serve as the basis for modification, amendment, or extension, by implication or otherwise, of his employment with the Company. However, as described in this Agreement, Executive may be entitled to severance benefits depending on the circumstances of Executive’s termination of employment with the Company.

3. Term of Agreement. Subject to Section 2 above, this Agreement will have a term of three (3) years, commencing on the Effective Date (the “**Employment Term**”). At the end of the Employment Term, the Agreement may be renewed upon mutual agreement in writing by Executive and the Company, otherwise it will expire in accordance with its terms. The Company and Executive agree to discuss the renewal of the Agreement not less than six (6) months prior to the end of the Employment Term. Non-renewal at the end of the Employment Term shall not constitute termination without Cause or give Executive an opportunity to terminate his employment for Good Reason, even if a Good Reason event has occurred before the expiration of the Employment Term under this Agreement, *provided*, that in the event the Company terminates Executive’s employment without Cause in connection with such non-renewal, then Executive shall be entitled to the severance benefits set forth in Section 8 subject to the terms and conditions thereof. Notwithstanding anything herein to the contrary, if, during the Employment Term, the Company experiences a Change of Control, the Employment Term shall be extended to the end of the Change of Control Period (as defined in Section 8(b) below).

4. Compensation/Relocation Reimbursement.

(a) Base Salary. Effective January 1, 2013, Executive's annual base salary shall be \$580,000 (as adjusted from time to time, the "**Base Salary**"). The Base Salary will be paid periodically in accordance with the Company's normal payroll practices and be subject to the usual, required withholdings. Executive's Base Salary shall be reviewed by the Board or the Compensation Committee of the Board not less often than annually, and may be adjusted from time to time.

(b) Target Bonus. Executive will continue to be eligible to receive a target annual bonus of fifty percent (50%) of Executive's Base Salary, less applicable withholdings, upon achievement of performance objectives to be determined by the Board in its sole discretion (the "**Target Bonus**"). The maximum bonus Executive will be eligible to receive is one hundred fifty percent (150%) of his Base Salary. The Target Bonus, or any portion thereof, will be paid as soon as practicable after the Board determines that the Target Bonus has been earned, but in no event shall the Target Bonus be paid after the later of (i) the fifteenth (15th) day of the third (3rd) month following the close of the Company's fiscal year in which the Target Bonus is earned or (ii) March 15 following the calendar year in which the Target Bonus is earned.

(c) Equity Awards. Executive shall be eligible to be granted additional equity awards in accordance with the Company's policies as in effect from time to time.

(d) Relocation Reimbursement. In conjunction with completing the relocation of the corporate headquarters from the Seattle, Washington area to the greater Boston, MA metropolitan area in the first half of 2013 (the "**Relocation Period**"), the Company agrees to reimburse Executive for his actual, documented reasonable expenses incurred in moving and relocating to the Boston, MA metropolitan area up to a total of \$55,000, including any costs or expenses associated with Executive's (i) lease-break of current residence, (ii) shipment of personal effects to the Boston, MA metropolitan area (iii) temporary housing in the Boston, MA metropolitan area for three (3) months following the move, (iv) costs associated with storage of household goods in the Boston, MA metropolitan area for six (6) months following the move, as well as other miscellaneous expenses as defined in the standard relocation policy and employee guidelines governing the group move for employees who are re-locating from the Seattle area to the Boston, MA metropolitan area. The Company will make an additional payment to Executive in the amount necessary to make Executive whole for taxes incurred in connection with the reimbursement provided under this Section 4(d) as well as any taxable reimbursement for relocation expenses provided under the Prior Agreement. In addition, Executive agrees that he will submit all such reimbursable expenses to the Company with appropriate documentation no later than ninety (90) days after such expenses are incurred, and the Company shall reimburse Executive promptly thereafter in accordance with the Company's expense reimbursement policy. Notwithstanding anything in this Section 4(d) to the contrary, any payments or reimbursements pursuant to this Section 4(d) shall be made no later than March 14, 2014.

5. Employee Benefits. During the Employment Term, Executive will continue to be entitled to participate in the employee benefit plans currently and hereafter maintained by the

Company of general applicability to other executive officers of the Company. The Company reserves the right to cancel or change the benefit plans and programs it offers to its employees at any time.

6. Vacation. Executive will continue to be entitled to paid vacation in accordance with the Company's vacation policy, with the timing and duration of extended vacations communicated to the Company in advance.

7. Business Expenses. The Company will continue to reimburse Executive for reasonable travel, entertainment or other business expenses incurred by Executive in the furtherance of, or in connection with, the performance of Executive's duties hereunder, in accordance with the Company's expense reimbursement policy as in effect from time to time.

8. Severance.

(a) Termination for other than Cause, Death or Disability Apart from a Change of Control. If prior to a Change of Control or after twelve (12) months following a Change of Control, the Company (or any parent or subsidiary or successor of the Company) terminates Executive's employment with the Company other than for Cause (as defined below), death or disability, then, subject to Section 9, in addition to any accrued but unpaid salary, bonus, vacation and expense reimbursement payable in accordance with applicable law, Executive will be entitled to:

(i) receive continuing payments of severance pay at a rate equal to Executive's Base Salary, as then in effect, for twelve (12) months from the date of such termination, which will be paid, less applicable withholding obligations, in accordance with the Company's regular payroll procedures, such installments to commence on the first payroll date following the date the Release (as defined below) becomes effective and irrevocable;

(ii) each outstanding equity award, including, without limitation, each stock option and restricted stock award, held by Executive shall automatically become vested and, if applicable, exercisable and any forfeiture restrictions or rights of repurchase thereon shall immediately lapse, in each case, with respect to fifty percent (50%) of shares subject thereto; and

(iii) an extension of the post-termination exercise period applicable to Executive's outstanding options to purchase Company common stock to one hundred and eighty (180) days following the date of Executive's termination of employment.

(b) Termination for other than Cause, Death or Disability or Resignation by Executive for Good Reason upon or within Twelve Months Following a Change of Control. If upon or within twelve (12) months following a Change of Control (the "**Change of Control Period**"), the Company (or any parent or subsidiary or successor of the Company) terminates Executive's employment with the Company other than for Cause, death or disability or the Executive resigns from such employment for Good Reason (as defined below), then, subject to Section 9, in addition to any accrued but unpaid salary, bonus, vacation and expense reimbursement payable in accordance with applicable law, Executive will be entitled to:

(i) receive continuing payments of severance pay at a rate equal to Executive's Base Salary, as then in effect, for twenty-four (24) months from the date of such termination, which will be paid, less applicable withholding obligations, in accordance with the Company's regular payroll procedures, such installments to commence on the first payroll date following the date the Release (as defined below) becomes effective and irrevocable;

(ii) receive an amount equal to one hundred percent (100%) of Executive's Annual Target Bonus assuming achievement of performance goals at one hundred percent (100%) payable in a cash lump sum, less applicable withholdings, as soon as administratively practicable following the date the Release is not subject to revocation and, in any event, within sixty (60) days following the date of termination;

(iii) each outstanding equity award, including, without limitation, each stock option and restricted stock award, held by Executive shall automatically become vested and, if applicable, exercisable and any forfeiture restrictions or rights of repurchase thereon shall immediately lapse, in each case, with respect to one hundred percent (100%) of shares subject thereto;

(iv) an extension of the post-termination exercise period applicable to Executive's outstanding options to purchase Company common stock to one hundred and eighty (180) days following the date of Executive's termination of employment; and

(v) if Executive elects to receive continued healthcare coverage pursuant to the provisions of the Consolidated Omnibus Budget Reconciliation Act of 1985, as amended ("COBRA"), the Company shall directly pay, or reimburse Executive for, the premium for Executive and Executive's covered dependents through the earlier of (i) the twenty-four (24) month anniversary of the date of Executive's termination of employment and (ii) the date Executive and Executive's covered dependents, if any, become eligible for healthcare coverage under another employer's plan(s). After the Company ceases to pay premiums pursuant to the preceding sentence, Executive may, if eligible, elect to continue healthcare coverage at Executive's expense in accordance the provisions of COBRA.

(c) Termination for Cause, Death or Disability; Resignation without Good Reason. If Executive's employment with the Company (or any parent or subsidiary or successor of the Company) terminates voluntarily by Executive (except upon resignation for Good Reason during the Change of Control Period), for Cause by the Company or due to Executive's death or disability, then

(i) all vesting with respect to each outstanding equity award, including, without limitation, each stock option and restricted stock award, held by Executive shall automatically terminate, and all such unvested awards shall be of no further force or effect;

(ii) all payments of compensation by the Company to Executive hereunder will terminate immediately (except as to amounts already earned);

(iii) Executive shall receive any accrued but unpaid salary, bonus, vacation and expense reimbursement payable in accordance with applicable law, and

(iv) Executive will only be eligible for severance benefits in accordance with applicable law and the Company's established policies, if any, as then in effect.

(d) Exclusive Remedy. In the event of a termination of Executive's employment with the Company (or any parent or subsidiary or successor of the Company), the provisions of this Section 8 are intended to be and are exclusive and in lieu of any other rights or remedies to which Executive or the Company may otherwise be entitled, whether at law, tort or contract, in equity, or under this Agreement. Executive will be entitled to no severance or other benefits upon termination of employment with respect to acceleration of award vesting, extension of the option exercise period, or severance pay other than those benefits expressly set forth in this Section 8.

9. Conditions to Receipt of Severance; No Duty to Mitigate.

(a) Separation Agreement and Release of Claims. The receipt of any severance pursuant to Section 8(a) or (b) will be subject to Executive signing and not revoking a separation agreement and release of claims in a form reasonably satisfactory to the Company (the "**Release**") and provided that such Release becomes effective and irrevocable no later than sixty (60) days following the termination date (such deadline, the "**Release Deadline**"). No severance will be paid or provided until the Release becomes effective and irrevocable. If the Release does not become effective and irrevocable by the Release Deadline, Executive forfeits his right to any severance or similar payment under the Agreement. In the event Executive's termination of employment occurs at a time during the calendar year where it would be possible for the Release to become effective in the calendar year following the calendar year in which his termination of employment occurs, then any severance that would be deferred in accordance with the paragraph below will be paid on the first payroll date to occur during the calendar year following the calendar year in which such termination of employment occurs, or such later time as required by (i) the payment schedule applicable to each payment or benefit, (ii) the date the Release becomes effective, or (iii) Section 9(c) below.

(b) Non-Competition; Non-Solicitation. The receipt of any severance benefits pursuant to Section 8(a) or (b) will be subject to Executive not violating the provisions of Sections 13 and 14. In the event Executive breaches the provisions of Sections 13 and/or 14, or otherwise materially breaches this Agreement, all continuing payments and benefits to which Executive may otherwise be entitled pursuant to Section 8(a) or (b), as applicable, will immediately cease.

(c) Section 409A.

(i) Notwithstanding anything to the contrary in this Agreement, no severance pay or benefits to be paid or provided to Executive, if any, pursuant to this Agreement that, when considered together with any other severance payments or separation benefits, are considered deferred compensation under Code Section 409A, and the final regulations and any

guidance promulgated thereunder (“**Section 409A**”) (together, the “**Deferred Payments**”) will be paid or otherwise provided until Executive has a “separation from service” within the meaning of Section 409A. Similarly, no severance payable to Executive, if any, pursuant to this Agreement that otherwise would be exempt from Section 409A pursuant to Treasury Regulation Section 1.409A-1(b)(9) will be payable until Executive has a “separation from service” within the meaning of Section 409A.

(ii) Any severance payments or benefits under this Agreement that would be considered Deferred Payments will be paid on, or, in the case of installments, will not commence until, the sixtieth (60th) day following Executive’s separation from service, or, if later, such time as required by Section 9(c)(iii). Except as required by Section 9(c)(iii), any installment payments that would have been made to Executive during the sixty (60) day period immediately following Executive’s separation from service but for the preceding sentence will be paid to Executive on the sixtieth (60th) day following Executive’s separation from service and the remaining payments shall be made as provided in this Agreement.

(iii) Notwithstanding anything to the contrary in this Agreement, if Executive is a “specified employee” within the meaning of Section 409A at the time of Executive’s termination (other than due to death), then the Deferred Payments that are payable within the first six (6) months following Executive’s separation from service, will become payable on the first payroll date that occurs on or after the date six (6) months and one (1) day following the date of Executive’s separation from service. All subsequent Deferred Payments, if any, will be payable in accordance with the payment schedule applicable to each payment or benefit. Notwithstanding anything herein to the contrary, if Executive dies following Executive’s separation from service, but prior to the six (6) month anniversary of the separation from service, then any payments delayed in accordance with this paragraph will be payable in a lump sum as soon as administratively practicable after the date of Executive’s death and all other Deferred Payments will be payable in accordance with the payment schedule applicable to each payment or benefit. Each payment and benefit, including, without limitation, any installment payments, payable under this Agreement is intended to constitute a separate payment for purposes of Section 1.409A-2(b)(2) of the Treasury Regulations.

(iv) Any amount paid under this Agreement that satisfies the requirements of the “short-term deferral” rule set forth in Section 1.409A-1(b)(4) of the Treasury Regulations will not constitute Deferred Payments for purposes of clause (i) above.

(v) Any amount paid under this Agreement that qualifies as a payment made as a result of an involuntary separation from service pursuant to Section 1.409A-1(b)(9)(iii) of the Treasury Regulations that does not exceed the Section 409A Limit (as defined below) will not constitute Deferred Payments for purposes of clause (i) above.

(vi) The foregoing provisions are intended to comply with the requirements of Section 409A so that none of the severance payments and benefits to be provided hereunder will be subject to the additional tax imposed under Section 409A, and any ambiguities herein will be interpreted to so comply. The Company and Executive agree to work together in good faith to consider amendments to this Agreement and to take such reasonable

actions which are necessary, appropriate or desirable to avoid imposition of any additional tax or income recognition prior to actual payment to Executive under Section 409A.

(d) Confidential Information Agreement. Executive's receipt of any payments or benefits under Section 8 will be subject to Executive continuing to comply with the terms of Confidential Information Agreement (as defined in Section 12).

(e) No Duty to Mitigate. Executive will not be required to mitigate the amount of any payment contemplated by this Agreement, nor will any earnings that Executive may receive from any other source reduce any such payment.

10. Definitions.

(a) Cause. For purposes of this Agreement, "**Cause**" is defined as (i) an act of dishonesty made by Executive in connection with Executive's responsibilities as an employee, (ii) Executive's conviction of, or plea of nolo contendere to, a felony or any crime involving fraud, embezzlement or any other act of moral turpitude; (iii) Executive's gross misconduct; (iv) Executive's unauthorized use or disclosure of any proprietary information or trade secrets of the Company or any other party to whom Executive owes an obligation of nondisclosure as a result of Executive's relationship with the Company; (v) Executive's willful breach of any obligations under any written agreement or covenant with the Company; or (vi) Executive's continued failure to perform his employment duties after Executive has received a written demand of performance from the Company which specifically sets forth the factual basis for the Company's belief that Executive has not substantially performed his duties and has failed to cure such non-performance to the Company's satisfaction within ten (10) business days after receiving such notice.

(b) Change of Control. For purposes of this Agreement, "**Change of Control**" of the Company is defined as:

(i) any "person" (as such term is used in Sections 13(d) and 14(d) of the Securities Exchange Act of 1934, as amended) is or becomes the "beneficial owner" (as defined in Rule 13d-3 under said Act), directly or indirectly, of securities of the Company representing more than fifty percent (50%) of the total voting power represented by the Company's then outstanding voting securities; or

(ii) the date of the consummation of a merger or consolidation of the Company with any other corporation that has been approved by the stockholders of the Company, other than a merger or consolidation which would result in the voting securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or its parent) at least fifty percent (50%) of the total voting power represented by the voting securities of the Company or such surviving entity or its parent outstanding immediately after such merger or consolidation; or

(iii) the date of the consummation of the sale or disposition by the Company of all or substantially all the Company's assets.

Notwithstanding the foregoing provisions of this definition, a transaction will not be deemed a Change of Control unless the transaction qualifies as a “change in control event” within the meaning of Section 409A.

(c) Code. For purposes of this Agreement, “**Code**” means the Internal Revenue Code of 1986, as amended.

(d) Good Reason. For the purposes of this Agreement, “**Good Reason**” means the termination by Executive upon the occurrence of any of the below described events. Executive must provide notice to the Company of the existence of such event within ninety (90) days of the first occurrence of such event, and the Company will have thirty (30) days to remedy the condition, in which case no Good Reason shall exist. If the Company fails to remedy the condition within such thirty (30) day period, Executive must terminate employment within two (2) years of the first occurrence of such event. The events which constitute a Good Reason termination are: (i) the assignment of a different title or change that results in a material reduction in Executive’s duties or responsibilities; (ii) a material reduction by the Company in Executive’s base compensation, other than a reduction in his Base Salary that is part of a general salary reduction affecting employees generally and provided the reduction is not greater, percentage-wise, than the reduction affecting other employees generally or failure to provide an annual increase in base compensation commensurate with other executives; *provided, however*, in determining whether to provide an annual increase in base compensation commensurate with an annual increase provided to other executives, the Company may take into account factors such as market levels of compensation, Executive’s overall performance, and other factors reasonably considered by the Company’s compensation committee and/or Board, so long as such determination is not made in bad faith with the intent to discriminate against Executive; or (iii) relocation of Executive’s principal place of business of greater than seventy-five (75) miles from its then location.

(e) Section 409A Limit. For purposes of this Agreement, “**Section 409A Limit**” will mean two (2) times the lesser of: (i) Executive’s annualized compensation based upon the annual rate of pay paid to Executive during the Executive’s taxable year preceding the Executive’s taxable year of his or her separation from service as determined under Treasury Regulation Section 1.409A-1(b)(9)(iii)(A)(1) and any Internal Revenue Service guidance issued with respect thereto; or (ii) the maximum amount that may be taken into account under a qualified plan pursuant to Section 401(a)(17) of the Internal Revenue Code for the year in which Executive’s separation from service occurred.

11. Limitation on Payments. In the event that the severance and other benefits provided for in this Agreement or otherwise payable to Executive (i) constitute “parachute payments” within the meaning of Section 280G of the Code and (ii) but for this Section 11, would be subject to the excise tax imposed by Section 4999 of the Code, then Executive’s severance benefits will be either:

(a) delivered in full, or

(b) delivered as to such lesser extent which would result in no portion of such severance benefits being subject to the excise tax under Section 4999 of the Code,

whichever of the foregoing amounts, taking into account the applicable federal, state and local income taxes and the excise tax imposed by Section 4999, results in the receipt by Executive on an after-tax basis, of the greatest amount of severance benefits, notwithstanding that all or some portion of such severance benefits may be taxable under Section 4999 of the Code. If a reduction in the severance and other benefits constituting "parachute payments" is necessary so that no portion of such severance benefits is subject to the excise tax under Section 4999 of the Code, the reduction shall occur in the following order:

(1) reduction of the cash severance payments; (2) cancellation of accelerated vesting of equity awards; and (3) reduction of continued employee benefits. In the event that acceleration of vesting of equity award compensation is to be reduced, such acceleration of vesting shall be cancelled in the reverse order of the date of grant of Executive's equity awards. If two or more equity awards are granted on the same date, each award will be reduced on a pro-rata basis. In no event shall the Executive have any discretion with respect to the ordering of payment reductions.

Unless the Company and Executive otherwise agree in writing, any determination required under this Section 11 will be made in writing by the independent public accountants who are primarily used by the Company immediately prior to the Change of Control, the Company's legal counsel or such other person or entity to which the parties mutually agree (the "**Firm**"), whose determination will be conclusive and binding upon Executive and the Company for all purposes. For purposes of making the calculations required by this Section 11, the Firm may make reasonable assumptions and approximations concerning applicable taxes and may rely on reasonable, good faith interpretations concerning the application of Sections 280G and 4999 of the Code. The Company and Executive will furnish to the Firm such information and documents as the Firm may reasonably request in order to make a determination under this Section 11. The Company will bear all costs the Firm may reasonably incur in connection with any calculations contemplated by this Section 11.

12. Confidential Information. Executive agrees to continue to abide by the terms and conditions of that certain confidential information agreement by and between Executive and the Company (the "**Confidential Information Agreement**").

13. Non-Competition. During the term of Executive's employment with the Company and until the later of: the date Executive terminates his employment with the Company or the date Executive no longer receives the severance benefits provided in Section 8(a)(i) or 8(b)(i), as applicable, Executive will not, either directly or indirectly, (a) serve as an advisor, agent, consultant, director, employee, officer, partner, proprietor or otherwise of, (b) have any ownership interest in (except for passive ownership of one percent (1%) or less of any entity whose securities have been registered under the Securities Act of 1933, as amended, or Section 12 of the Securities Exchange Act of 1934, as amended) or (c) participate in the organization, financing, operation, management or control of, any business (i) that is in competition with the Company's business as conducted by the Company at any time during the course of Executive's employment with the Company and (ii) on which Executive worked or about which Executive learned, during his employment, information or knowledge not generally known or available

outside the Company, or information or physical material entrusted to the Company by third parties, including, but not limited to inventions, during Executive's employment or consultancy with the Company, confidential knowledge, copyrights, product ideas, techniques, processes, formulas, object codes, biological materials, mask works and/or any other information of any type relating to documentation, laboratory notebooks, data, schematics, algorithms, flow charts, mechanisms, research, manufacture, improvements, assembly, installation, marketing, forecasts, sales, pricing, customers, the salaries, duties, qualifications, performance levels and terms of compensation of other employees, and/or cost or other financial data concerning any of the foregoing or the Company and its operations.

14. Non-Solicitation. During the term of Executive's employment with the Company and until the date two (2) years after the termination of Executive's employment with the Company for any reason, Executive agrees not, either directly or indirectly, to solicit, induce, attempt to solicit, recruit, or encourage any employee of the Company (or any parent or subsidiary of the Company) to leave his employment either for Executive or for any other entity or person. Executive represents that he (a) is familiar with the foregoing covenant not to solicit, and (b) is fully aware of his obligations hereunder, including, without limitation, the reasonableness of the length of time, scope and geographic coverage of these covenants.

15. Assignment. This Agreement will be binding upon and inure to the benefit of (a) the heirs, executors and legal representatives of Executive upon Executive's death and (b) any successor of the Company. Any such successor of the Company will be deemed substituted for the Company under the terms of this Agreement for all purposes. For this purpose, "successor" means any person, firm, corporation or other business entity which at any time, whether by purchase, merger or otherwise, directly or indirectly acquires all or substantially all of the assets or business of the Company. None of the rights of Executive to receive any form of compensation payable pursuant to this Agreement may be assigned or transferred except by will or the laws of descent and distribution. Any other attempted assignment, transfer, conveyance or other disposition of Executive's right to compensation or other benefits will be null and void.

16. Notices. All notices, requests, demands and other communications called for hereunder will be in writing and will be deemed given (a) on the date of delivery if delivered personally, (b) one (1) day after being sent by a well established commercial overnight service, or (c) four (4) days after being mailed by registered or certified mail, return receipt requested, prepaid and addressed to the parties or their successors at the following addresses, or at such other addresses as the parties may later designate in writing:

If to the Company:

Sarepta Therapeutics, Inc.
Attn: Chairman of the Board of Directors
245 First Street
Suite 1800
Cambridge, MA 02142

If to Executive:

at the last residential address known by the Company.

17. Severability. In the event that any provision hereof becomes or is declared by a court of competent jurisdiction to be illegal, unenforceable or void, this Agreement will continue in full force and effect without said provision.

18. Arbitration.

(a) General. In consideration of Executive's service to the Company, his/her promise to arbitrate all employment related disputes and Executive's receipt of the compensation, pay raises and other benefits paid to Executive by the Company, at present and in the future, Executive agrees that any and all controversies, claims, or disputes with anyone (including the Company and any employee, officer, director, stockholder or benefit plan of the Company in their capacity as such or otherwise) arising out of, relating to, or resulting from Executive's service to the Company under this Agreement or otherwise or the termination of Executive's service with the Company, including any breach of this Agreement, shall be subject to binding arbitration pursuant to Massachusetts law (the "**Rules**"). Disputes which Executive agrees to arbitrate, and thereby agrees to waive any right to a trial by jury, include any statutory claims under state or federal law, including, but not limited to, claims under Title VII of the Civil Rights Act of 1964, the Americans with Disabilities Act of 1990, the Age Discrimination in Employment Act of 1967, the Older Workers Benefit Protection Act, claims of harassment, discrimination or wrongful termination. Executive further understands that this Agreement to arbitrate also applies to any disputes that the Company may have with Executive.

(b) Procedure. Executive agrees that any arbitration will be administered by the American Arbitration Association ("**AAA**") and that a neutral arbitrator will be selected in a manner consistent with its National Rules for the Resolution of Employment Disputes. The arbitration proceedings will allow for discovery according to the rules set forth in the National Rules for the Resolution of Employment Disputes or the Massachusetts Rules of Civil Procedure. Executive agrees that the arbitrator shall have the power to decide any motions brought by any party to the arbitration, including motions for summary judgment and/or adjudication and motions to dismiss and demurrers, prior to any arbitration hearing. Executive agrees that the arbitrator shall issue a written decision on the merits with findings of fact and conclusions of law. Executive also agrees that the arbitrator shall have the power to award any remedies, including attorneys' fees and costs, available under applicable law. Executive understands the Company will pay for any administrative or hearing fees charged by the arbitrator or AAA except that, for any filing fees associated with any arbitration Executive initiates, Executive shall pay an amount equal to the filing fees Executive would have paid had he/she filed a complaint in a court of law. Executive agrees that the arbitrator shall administer and conduct any arbitration in a manner consistent with the Rules and that to the extent that the AAA's National Rules for the Resolution of Employment Disputes conflict with the Rules, the Rules shall take precedence.

(c) Remedy. Except as provided by the Rules, arbitration shall be the sole, exclusive and final remedy for any dispute between Executive and the Company. Accordingly,

except as provided for by the Rules, neither Executive nor the Company will be permitted to pursue court action regarding claims that are subject to arbitration. Notwithstanding, the arbitrator will not have the authority to disregard or refuse to enforce any lawful Company policy, and the arbitrator shall not order or require the Company to adopt a policy not otherwise required by law which the Company has not adopted.

(d) Availability of Injunctive Relief. In addition to the right under the Rules to petition the court for provisional relief, Executive agrees that any party may also petition the court for injunctive relief where either party alleges or claims a violation of this Agreement or the Confidential Information Agreement or any other agreement regarding trade secrets, confidential information, non-competition, non-solicitation or non-disparagement. In the event either party seeks injunctive relief, the prevailing party shall be entitled to recover reasonable costs and attorneys' fees.

(e) Administrative Relief. Executive understands that this Agreement does not prohibit Executive from pursuing an administrative claim with a local, state or federal administrative body such as the Equal Employment Opportunity Commission or the workers' compensation board. This Agreement does, however, preclude Executive from pursuing court action regarding any such claim.

(f) Voluntary Nature of Agreement. Executive acknowledges and agrees that Executive is executing this Agreement voluntarily and without any duress or undue influence by the Company or anyone else. Executive further acknowledges and agrees that Executive has carefully read this Agreement and that Executive has asked any questions needed for Executive to understand the terms, consequences and binding effect of this Agreement and fully understands it, including that ***EXECUTIVE IS WAIVING EXECUTIVE'S RIGHT TO A JURY TRIAL***. Finally, Executive agrees that Executive has been provided an opportunity to seek the advice of an attorney of Executive's choice before signing this Agreement.

19. Integration. This Agreement, together with the Confidential Information Agreement and the equity award agreements by and between Executive and the Company, represents the entire agreement and understanding between the parties as to the subject matter herein and supersedes all prior or contemporaneous agreements whether written or oral, including, without limitation, the Prior Agreement. With respect to stock options or other equity awards granted on or after the date of this Agreement, the acceleration of vesting provisions provided herein will apply to such stock options and other equity awards except to the extent otherwise explicitly provided in the applicable stock option or equity award agreement. This Agreement may be modified only by agreement of the parties by a written instrument executed by the parties that is designated as an amendment to this Agreement.

20. Waiver of Breach. The waiver of a breach of any term or provision of this Agreement, which must be in writing, will not operate as or be construed to be a waiver of any other previous or subsequent breach of this Agreement.

21. Headings. All captions and section headings used in this Agreement are for convenient reference only and do not form a part of this Agreement.

22. Tax Withholding. All payments made pursuant to this Agreement will be subject to withholding of applicable taxes.

23. Governing Law. This Agreement will be governed by the laws of the Commonwealth of Massachusetts (with the exception of its conflict of laws provisions).

24. Acknowledgment. Executive acknowledges that he has had the opportunity to discuss this matter with and obtain advice from his private attorney, has had sufficient time to, and has carefully read and fully understands all the provisions of this Agreement, and is knowingly and voluntarily entering into this Agreement.

25. Counterparts. This Agreement may be executed in counterparts, and each counterpart will have the same force and effect as an original and will constitute an effective, binding agreement on the part of each of the undersigned.

IN WITNESS WHEREOF, each of the parties has executed this Agreement, in the case of the Company by their duly authorized officers, as of the day and year first above written.

EXECUTIVE

/s/ Chris Garabedian
Chris Garabedian

April 19, 2013
Date

SAREPTA THERAPEUTICS, INC.

/s/ Sandesh Mahatme
By: Sandesh Mahatme
Title: Chief Financial Officer

April 19, 2013
Date

[SIGNATURE PAGE TO EXECUTIVE EMPLOYMENT AGREEMENT]

May 9, 2013

Michael A. Jacobsen
18515 28th Ave S.E.
Bothell, WA 98012

Re: **Retention and Severance Benefits**

Dear Mike:

As we have recently discussed, the Compensation Committee (the "Committee") of the Board of Directors of Sarepta Therapeutics, Inc. (the "Company") approved a retention and severance benefits package for you. This letter summarizes the benefits approved by the Committee and sets forth the terms and conditions for you to receive the approved benefits.

Provided that you do not resign, for any reason whatsoever, from your employment with the Company on or before May 31, 2013, then the Company will terminate your employment effective May 31, 2013 (the "Termination Date") and provide you with the following:

- (i) On August 23, 2012, you were awarded 7,500 restricted stock units (the "RSUs") under the AVI BioPharma, Inc. 2011 Equity Incentive Plan (the "Plan"). All of the RSUs granted to you on August 23, 2012 under the Plan that remain unvested on the Termination Date shall immediately vest on the Termination Date;
- (ii) On April 24, 2012, you were awarded an option to purchase 3,702 shares of Company common stock (after giving effect to the Company's reverse stock split) (the "Option") under the Plan. All unvested shares of Company common stock granted to you as an Option on April 24, 2012 under the Plan that remain unvested on the Termination Date shall immediately vest on the Termination Date and shall be exercisable in accordance with the terms of the Plan;
- (iii) Provided you first execute and deliver, and do not revoke, a General Release of Claims in a form reasonably acceptable to the Company (the "General Release"), which execution must be on June 1, 2013 or later, the Company will provide you with a lump sum severance payment in the amount of Seventy Eight Thousand Five Hundred and Fifty Four Dollars and Sixty Six Cents (\$78,554.66; the "Severance Pay"), which amount is approximately equivalent to eight weeks of your base salary and a pro-rated 2013 bonus at 100% of target. Such Severance Pay shall be provided to you within thirty (30) calendar days after the expiration of the revocation period set forth in the General Release; and
- (iv) If you timely complete the election forms (which will be provided to you separately) under the Consolidated Omnibus Budget Reconciliation Act of 1985 ("COBRA") to continue receiving group medical and/or dental insurance, the Company will reimburse you for 100% of the associated COBRA premiums for a period of one (1) month. Thereafter, you may, at your own expense, continue

participation in COBRA for the balance of time provided by the statute; provided, however, that the “qualifying event” under COBRA shall be deemed to have occurred on the Termination Date.

This letter agreement — together with the Plan; the agreement evidencing the August 23, 2012 Company grant of RSUs to you; the agreement evidencing the April 24, 2012 Company Option grant to you; and the documents evidencing your 2013 Target Bonus — constitute the entire understanding of the parties with respect to the subject matter hereof and supersede all prior arrangements and understandings regarding such subject matter, including, without limitation, any offer letter agreement and/or employment agreement.

To acknowledge the retention and severance benefits described herein, please sign and return a copy of this letter agreement to Ty Howton by email or by fax to 617-812-5811 by 2 p.m. EST, May 9, 2013.

SAREPTA THERAPEUTICS, INC.

By: /s/ David Tyrone Howton
Name: David Tyrone Howton
Title: Senior Vice President, General Counsel
and Corporate Secretary

ACKNOWLEDGED AND AGREED

/s/ Michael A. Jacobsen

Michael A. Jacobsen

Date: May 9, 2013

CERTIFICATION

I, Christopher Garabedian, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Registrant as of, and for, the periods presented in this report;

4. The Registrant’s other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the Registrant’s disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the Registrant’s internal control over financial reporting that occurred during the Registrant’s most recent fiscal quarter (the Registrant’s fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the Registrant’s internal control over financial reporting; and

5. The Registrant’s other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Registrant’s auditors and the audit committee of the Registrant’s board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Registrant’s ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the Registrant’s internal control over financial reporting.

May 9, 2013

/s/ Christopher Garabedian

Christopher Garabedian
President and Chief Executive Officer
(Principal Executive Officer)

CERTIFICATION

I, Sandesh Mahatme, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Registrant as of, and for, the periods presented in this report;

4. The Registrant’s other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the Registrant’s disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the Registrant’s internal control over financial reporting that occurred during the Registrant’s most recent fiscal quarter (the Registrant’s fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the Registrant’s internal control over financial reporting; and

5. The Registrant’s other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Registrant’s auditors and the audit committee of the Registrant’s board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Registrant’s ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the Registrant’s internal control over financial reporting.

May 9, 2013

/s/ Sandesh Mahatme

Sandesh Mahatme
Senior Vice President, Chief Financial Officer
(Principal Financial Officer)

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

I, Christopher Garabedian, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended March 31, 2013, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

May 9, 2013

/s/ Christopher Garabedian

Christopher Garabedian,
President and Chief Executive Officer
(Principal Executive Officer)

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

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

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

I, Sandesh Mahatme, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended March 31, 2013, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

May 9, 2013

/s/ Sandesh Mahatme

Sandesh Mahatme
*Senior Vice President, Chief Financial Officer
(Principal Financial Officer)*

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

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

