Sarepta Therapeutics, Inc.
Form 10-Q
November 05, 2015

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

x QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended September 30, 2015

OR

o TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to

Commission file number 001-14895

SAREPTA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

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

(Address of principal executive offices) (Zip Code)
Registrant's telephone number, including area code: (617) 274-4000

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

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

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

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

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

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

Common Stock with $0.0001 par value	45,271,301
(Class)	(Outstanding as of October 30, 2015)

SAREPTA THERAPEUTICS, INC.

FORM 10-Q

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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(unaudited, in thousands, except per share amounts)

	As of September 30, 2015	As of December 31, 2014
Assets		
Current Assets:		
Cash and cash equivalents	$55,819	$73,551
Short-term investments	44,090	136,793
Accounts receivable	2,733	2,416
Other current assets	19,466	35,036
Total Current Assets	122,108	247,796
Restricted cash and investments	11,478	782
Property and equipment, net of accumulated depreciation of $23,368 and $19,896 as of September 30, 2015 and December 31, 2014, respectively	37,379	38,501
Patent costs, net of accumulated amortization of $2,486 and $2,081 as of September 30, 2015 and December 31, 2014, respectively	6,116	5,891
Other assets	7,670	2,063
Total Assets	$184,751	$295,033
Liabilities and Stockholders' Equity		
Current Liabilities:		
Accounts payable	$7,431	$12,408
Accrued expenses	19,761	17,366
Current portion of long-term debt	3,435	98
Current portion of notes payable	2,454	2,492
Deferred revenue	3,303	3,318
Other current liabilities	1,283	1,185
Total Current Liabilities	37,667	36,867
Long-term debt	17,397	1,476

Notes payable	—	2,262
Deferred rent and other	6,451	6,775
Total Liabilities	61,515	47,380
Commitments and contingencies		
Stockholders' Equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $.0001 par value, 99,000,000 shares authorized; 41,963,151 and 41,311,512 issued and outstanding at September 30, 2015 and December 31, 2014, respectively	4	4
Additional paid-in capital	957,607	926,769
Accumulated other comprehensive loss	(1)	(95)
Accumulated deficit	(834,374)	(679,025)
Total Stockholders' Equity	123,236	247,653
Total Liabilities and Stockholders' Equity	$184,751	$295,033

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited, in thousands, except per share amounts)

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2015	2014	2015	2014
Revenue from research contracts and other grants	$ —	$ 1,059	$ —	$ 9,730
Operating expenses:				
Research and development	36,673	21,852	105,018	63,399
General and administrative	15,090	12,882	50,714	35,398
Total operating expenses	51,763	34,734	155,732	98,797
Operating loss	(51,763)	(33,675)	(155,732)	(89,067)
Other income (loss):				
Interest (expense) income and other, net	(176)	193	383	473
Gain (loss) on change in warrant valuation	—	4,256	—	(2,779)
Total other (loss) income	(176)	4,449	383	(2,306)
Net loss	$ (51,939)	$ (29,226)	$ (155,349)	$ (91,373)
Other comprehensive income (loss):				
Unrealized gain (loss) on short-term securities - available-for-sale	18	(21)	94	(55)
Total other comprehensive income (loss)	18	(21)	94	(55)
Comprehensive loss	$ (51,921)	$ (29,247)	$ (155,255)	$ (91,428)
Net loss per share — basic and diluted	$ (1.25)	$ (0.71)	$ (3.75)	$ (2.31)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	41,565	41,066	41,416	39,595

See accompanying notes to unaudited condensed consolidated financial statements.

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SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited, in thousands)

	For the Nine Months Ended September 30,	
	2015	2014
Cash flows from operating activities:		
Net loss	$ (155,349)	$ (91,373)
Adjustments to reconcile net income to cash flows in operating activities:		
Depreciation and amortization	3,883	2,532
Amortization of premium on available-for-sale securities and non-cash interest	805	1,848
Loss on abandonment of patents	180	52
Stock-based compensation	25,769	14,578
Increase in warrant valuation	—	2,779
Changes in operating assets and liabilities, net:		
Net (increase) decrease in accounts receivable	(317)	168
Net decrease (increase) in other assets	9,963	(29,168)
Net decrease in accounts payable, accrued expenses, deferred revenue and other liabilities	(3,127)	(4,506)
Net cash used in operations	(118,193)	(103,090)
Cash flows from investing activities:		
Release and maturity of restricted investments	—	3,250
Purchase of restricted investments	(10,695)	—
Purchase of property and equipment	(2,316)	(22,305)
Patent costs	(982)	(1,062)
Purchase of available-for-sale securities	(49,632)	(272,189)
Maturity of available-for-sale securities	141,854	86,599
Net cash from (used in) investing activities	78,229	(205,707)
Cash flows from financing activities:		
Proceeds from borrowings, net of debt issuance costs	19,601	—
Repayments of long-term debt and notes payable	(2,573)	(70)
Proceeds from exercise of options and warrants and the sale of common stock, net of offering costs	5,204	104,201
Net cash from financing activities	22,232	104,131
Decrease in cash and cash equivalents	(17,732)	(204,666)
Cash and cash equivalents:		
Beginning of period	73,551	256,965
End of period	$ 55,819	$ 52,299

Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 359	$ 60
Supplemental schedule of non-cash investing activities and financing activities:		
Accrued debt issuance costs related to the senior secured term loan	$ 400	$ —
Property and equipment included in accrued expenses	$ 211	$ 1,165
Patent costs included in accrued expenses	$ 105	$ 187
Accrued legal and other fees for the October 9, 2015 common stock offering (See Note 11, Subsequent Event)	$ 135	$ —
Capitalized interest	$ 99	$ 36
Issuance of common stock in satisfaction of warrants	$ —	$ 11,785
Tenant improvement paid by Landlord	$ —	$ 154
Issuance of note payable in relation to the purchase of certain real and personal property located in Andover, Massachusetts	$ —	$ 4,613

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. BUSINESS AND BASIS OF PRESENTATION

Business

Sarepta Therapeutics, Inc. (together with its wholly-owned subsidiaries "Sarepta" or the "Company") is a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other diseases. Applying its proprietary, highly-differentiated and innovative platform technologies, the Company is able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy ("DMD") drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. On August 25, 2015, the Company announced the filing by the Food and Drug Administration ("FDA") of its new drug application ("NDA") for eteplirsen for the treatment of DMD amenable to exon 51 skipping. Eteplirsen is under priority review with a Prescription Drug User Fee Act ("PDUFA") action date of February 26, 2016. The Company is also developing therapeutics using its technology for the treatment of drug resistant bacteria and infectious, rare and other human diseases.

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

On October 9, 2015, the Company completed a public offering whereby the Company sold 3,250,000 shares of common stock at a price of $39.00 per share. In addition, the Company granted the underwriters a 30-day option to purchase an additional 487,500 shares of common stock at a price of $39.00 per share. There can be no assurance that the underwriters will exercise the option. The Company received aggregate net proceeds from the offering of approximately $120.0 million, after deducting the underwriting discounts and offering-related transaction costs.

As of September 30, 2015, the Company had approximately $111.4 million of cash, cash equivalents and investments, consisting of $55.8 million of cash and cash equivalents, $44.1 million of short-term investments and $11.5 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of September 30, 2015, together with the net proceeds of approximately $120.0 million received from the Company's common stock offering completed on October 9, 2015, is sufficient to fund its current operational plan for the next twelve months, though it may pursue additional cash resources through public or private financings, seek additional government contracts and establish collaborations with or license its technology to other companies.

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP"), reflect the accounts of Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries. All inter-company transactions between and among its

consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: the development of pharmaceutical products on its own behalf or in collaboration with others. The information included in this quarterly report on Form 10-Q should be read in conjunction with the Company's consolidated financial statements and the accompanying notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014.

Estimates and Uncertainties

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

2. RECENT ACCOUNTING PRONOUNCEMENTS

In April 2015, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2015-03, "Simplifying the Presentation of Debt Issuance Costs". The amendments in this update require that debt issuance costs related to a

recognized debt liability be presented in the balance sheet as a direct deduction from the carrying amount of that debt liability, consistent with debt discounts. ASU No. 2015-03 will be effective for fiscal years beginning after December 15, 2015, with early adoption permitted. The Company has elected to adopt this ASU early and the adoption of this guidance did not have a material effect on its consolidated financial statements. For additional information, please read Note 7, Long-term Debt of the unaudited condensed consolidated financial statements.

In August 2014, the FASB issued ASU No. 2014-15, “Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern”. This update requires an entity’s management to evaluate whether there are conditions or events, considered in the aggregate, that raise substantial doubt about the entity’s ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued and to provide related disclosures. ASU No. 2014-15 is effective for the annual period ending after December 15, 2016, with early adoption permitted. The Company has not adopted this guidance as of September 30, 2015, and based on the Company's financial condition as of the date these financial statements were issued or available for issuance, the Company does not expect the adoption of this guidance to have any impact on the current period financial statements.

In May 2014, the FASB issued ASU No. 2014-09, “Revenue from Contracts with Customers (Topic 606)”. This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, Revenue Recognition, and creates a new Topic 606, Revenue from Contracts with Customers. Under the new guidance, a company is required to recognize revenue when it transfers goods or renders services to customers at an amount that it expects to be entitled to in exchange for these goods or services. This guidance is effective for the fiscal years beginning after December 15, 2016, with early adoption not permitted. In August 2015, the FASB issued ASU No. 2015-14, “Deferral of the Effective Date”, which states that the mandatory effective date of this new revenue standard will be delayed by one year, with early adoption only permitted in fiscal year 2017. Two adoption methods are permitted: (i) retrospectively to all prior reporting periods presented, with certain practical expedients permitted; or (ii) retrospectively with the cumulative effect of initially adopting the ASU recognized at the date of initial application. The Company has not yet determined which adoption method it will utilize or the effect that the adoption of this guidance will have on its consolidated financial statements.

3. FAIR VALUE MEASUREMENTS

The Company has certain financial assets that are recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements.

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

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

	Fair Value Measurement as of September 30, 2015			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$140	$140	$—	$ —
Commercial paper	11,099	—	11,099	—
Government and government agency bonds	28,249	—	28,249	—
Corporate bonds	15,841	—	15,841	—
Certificates of deposit	11,343	11,343	—	—
Total assets	$66,672	$11,483	$55,189	$ —

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

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds and certificates of deposit. Money market funds are publicly traded mutual funds and are presented as cash equivalents on the unaudited condensed consolidated balance sheets as of September 30, 2015.

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

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

4. CASH, CASH EQUIVALENTS AND SHORT-TERM INVESTMENTS

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. The weighted average maturity of the Company's available-for-sale securities as of September 30, 2015 and December 31, 2014 was less than 1 and 4 months, respectively.

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

	As of September 30, 2015			
	Amortized Cost	Gross Unrealized	Gross Unrealized	Fair Market

		Gains	Losses	Value
	(in thousands)			
Cash and money market funds	$44,720	$ —	$ —	$44,720
Commercial paper	11,099	—	—	11,099
Government and government agency bonds	28,248	1	—	28,249
Corporate bonds	15,843	—	(2)	15,841
Total assets	$99,910	$ 1	$ (2)	$99,909
As reported:				
Cash and cash equivalents	$55,819	$ —	$ —	$55,819
Short-term investments	44,091	1	(2)	44,090
Total assets	$99,910	$ 1	$ (2)	$99,909

	As of December 31, 2014			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$73,551	$ —	$ —	$73,551
Commercial paper	2,997	—	—	2,997
Government and government agency bonds	75,289	—	(39)	75,250
Corporate bonds	58,602	—	(56)	58,546
Total assets	$210,439	$ —	$ (95)	$210,344
As reported:				
Cash and cash equivalents	$73,551	$ —	$ —	$73,551
Short-term investments	136,888	—	(95)	136,793
Total assets	$210,439	$ —	$ (95)	$210,344

5. OTHER CURRENT ASSETS

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

	As of September 30, 2015	As of December 31, 2014
	(in thousands)	
Manufacturing-related deposits	$14,450	$ 30,668
Prepaid expenses	4,382	2,797
Other	634	1,571
Total other current assets	$19,466	$ 35,036

6. ACCRUED EXPENSES

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

	As of September 30, 2015	As of December 31, 2014
	(in thousands)	
Accrued employee compensation costs	$6,262	$ 6,170
Accrued clinical and preclinical costs	5,972	3,471
Accrued professional fees	3,349	3,403
Accrued contract manufacturing costs	2,145	3,271
Accrued research costs	565	311
Accrued facility-related costs	168	300
Other	1,300	440
Total accrued expenses	$19,761	$ 17,366

7. LONG-TERM DEBT

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

Borrowings under the Credit Agreement bear interest at a rate per annum equal to 7.75%, with only interest payments due through June 30, 2016. In addition to paying interest on the outstanding principal under the Credit Agreement, the Company will pay an origination fee equal to 0.50% of the amount of the term loan when advanced under the Credit Agreement, as well as a final payment fee equal to 2.00% of the amount borrowed under the Credit Agreement when the term loan is fully repaid. Commencing on

July 1, 2016 and continuing for the remaining twenty-four months of the facility, the Company will be required to make monthly principal payments of approximately $0.8 million, or $1.7 million if the facility is increased by the additional $20.0 million referenced above.

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

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

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

In connection with the senior secured term loan, the Company recorded $20.0 million as long-term debt in the unaudited condensed consolidated balance sheets as of September 30, 2015. In addition, the Company incurred approximately $0.8 million in debt issuance costs that were recorded as a direct deduction to the carrying value of the term loan in the unaudited condensed consolidated balance sheets. These costs are being amortized to interest expense using the effective interest method over the term of the loan. For the three and nine months ended September 30, 2015, the Company recognized $0.4 million and $0.5 million of interest expense related to the term loan, respectively.

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

	As of September 30, 2015 (in thousands)
Principal amount	$ 20,000
Unamortized debt issuance costs	(668)
Net carrying value of senior secured term loan	19,332
Other long-term debt	1,500
Total long-term debt	$ 20,832

8. STOCK-BASED COMPENSATION

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

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2015		2014		2015		2014	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options*	702,067	$ 24.05	237,050	$ 16.19	2,676,778	$ 14.67	1,540,085	$ 19.24
Restricted stock awards**	65,000	$ 33.81	—	$ —	181,783	$ 20.80	6,000	$ 29.03

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

** In September 2015, the Company granted certain employees restricted stock awards ("RSAs") with performance conditions. If certain performance milestones are achieved within the required time frame, the number of RSAs with performance conditions may be increased from 65,000 to 97,500 shares. As a result, the Company may recognize up to $3.3 million of stock-based compensation related to these grants when performance is deemed probable.

Stock-based Compensation Expense

For the three months ended September 30, 2015 and 2014, total stock-based compensation expense was $5.7 million and $4.6 million, respectively. For the nine months ended September 30, 2015 and 2014, total stock-based compensation expense was $25.8 million and $14.6 million, respectively. Included in the amount for the nine months ended September 30, 2015 is $8.6 million of stock-based compensation expense incurred in connection with the resignation of the Company's former Chief Executive Officer ("CEO"). The following table summarizes stock-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2015	2014	2015	2014
	(in thousands)			
Research and development	$2,631	$1,668	$7,639	$5,886
General and administrative	3,052	2,981	18,130	8,692
Total stock-based compensation expense	$5,683	$4,649	$25,769	$14,578

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

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2015	2014	2015	2014
	(in thousands)			
Stock options	$4,801	$4,217	$23,451	$13,170
Restricted stock awards	136	33	310	169
Restricted stock units	—	—	—	1
Stock appreciation rights	115	147	377	440
Employee stock purchase plan	631	252	1,631	798
Total stock-based compensation expense	$5,683	$4,649	$25,769	$14,578

9. NET LOSS PER SHARE

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

each of the periods presented, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and, therefore, would be excluded from the diluted net loss per share calculation.

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2015	2014	2015	2014
	(in thousands, except per share amounts)			
Net loss	$(51,939)	$(29,226)	$(155,349)	$(91,373)
Weighted-average number of shares of common stock and common stock equivalents outstanding:				
Weighted-average number of shares of common stock outstanding for computing basic loss per share	41,565	41,066	41,416	39,595
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	—	—	—
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	41,565	41,066	41,416	39,595
Net loss per share — basic and diluted	$(1.25)	$(0.71)	$(3.75)	$(2.31)

* For the three and nine months ended September 30, 2015 and 2014, stock options, RSAs and stock appreciation rights to purchase approximately 7.3 million and 5.3 million shares of common stock, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

10. COMMITMENTS AND CONTINGENCIES

Purchase Commitments

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

	As of September 30, 2015 (in thousands)
2015 (3 months)	$ 6,060
2016	57,047
2017	29,664
2018	28,521
2019	11,408
Total purchase commitments	$ 132,700

In connection with an amendment to a supply agreement, in September 2015, the Company issued an irrevocable standby letter of credit totaling $10.7 million to a contract manufacturing vendor. The obligation secured by the letter of credit will be fulfilled upon full payment of all deposits and purchase payments by the end of 2016. To meet the requirement of the letter of credit, the Company purchased $10.7 million in a certificate of deposit with a September 2016 maturity date. If the commitments have not occurred as of December 31, 2016, the letter of credit will be extended. The Company has recorded this $10.7 million as restricted investments in the unaudited condensed consolidated balance sheet as of September 30, 2015.

Litigation

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (Corban v. Sarepta, et. al., No. 14-cv-10201) by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. The plaintiffs' consolidated amended complaint, filed on July 21, 2014, sought to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleged that Sarepta and certain of its officers violated the federal securities laws in connection with disclosures related to eteplirsen, the Company's lead therapeutic candidate for DMD, and seeks damages in an unspecified amount. Pursuant to the court's June 23, 2014 order, Sarepta filed a motion to dismiss the consolidated amended complaint on August 18, 2014, and argument on the motion was held on March 12, 2015. On March 31, 2015, the Court dismissed plaintiffs' amended complaint. On

April 30, 2015, plaintiffs in the Corban suit filed a motion for leave seeking to file a further amended complaint, which the Company opposed. Following a hearing on August 12, 2015, the Court denied this motion, and on September 22, 2015, the Court dismissed the case. The plaintiffs filed a Notice of Appeal in the Court of Appeals for the First Circuit. No dates have been set for briefing or oral argument.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 by William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Christopher Garabedian, and Sandesh Mahatme (Kader v. Sarepta et.al 1:14-cv-14318), asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, Christopher Garabedian and Sandesh Mahatme. Plaintiffs' amended complaint, filed on March 20, 2015, alleges that the defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of an NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs seek compensatory damages and fees. The Company received service of the complaint on January 5, 2015. Sarepta filed a motion to dismiss the complaint on May 11, 2015, pursuant to the scheduling order entered on February 20, 2015, which plaintiffs have opposed. Oral argument on the motion to dismiss has not been scheduled.

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

obtained by the directors, and attorneys' fees. On March 24, 2015, the parties agreed to abate the case pending the resolution of both suits pending in federal court in the District of Massachusetts, Corban and Kader. Additionally, on February 24, 2015, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (Ira Gaines, and the Ira J. Gaines Revocable Trust U/A, on behalf of nominal defendant Sarepta Therapeutics, Inc., vs. Goolsbee et. al., No. 10713). The claims allege that the defendants participated in making material misrepresentations or omissions during the period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of the NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs seek unspecified compensatory damages, punitive damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. On March 26, 2015, the parties agreed to stay the case pending the resolution of Kader, pending in federal court in the District of Massachusetts.

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

11. SUBSEQUENT EVENT

Subsequent events have been evaluated through the date these consolidated financial statements were filed. On October 9, 2015, the Company sold 3,250,000 shares of common stock at a price of $39.00 per share. In addition, the Company granted the underwriters a 30-day option to purchase an additional 487,500 shares of common stock at a price of $39.00 per share. There can be no assurance that the underwriters will exercise the option. The Company received aggregate net proceeds of approximately $120.0 million, after deducting the underwriting discounts and offering-related transaction costs.

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

This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2014 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "estimate," "could," "continue," "ongoing," "predict," "pote and other similar expressions, as well as variations or negatives of these words. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state other "forward-looking" information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

- our expectations regarding the timing of research, development, preclinical and clinical trial results and analyses relating to the safety profile and potential clinical benefits of our product candidates, including eteplirsen, our phosphorodiamidate morpholino oligomer ("PMO") chemistries, our other PMO-based chemistries and our other RNA-targeted technologies;
- our expectations regarding additional data and analysis collected by us on eteplirsen;
- our expectations regarding the Food and Drug Administration's ("FDA") interpretation of our data and information on our product candidates, PMO and PMO-based chemistries and RNA-targeted technologies and the impact of the FDA's interpretations on our FDA submissions (including our investigational new drug applications ("INDs") and new drug applications ("NDAs")), filing decisions by the FDA, potential advisory committee meeting dates and advisory committee recommendations, and FDA product approval decisions and related timelines;
- our estimates regarding how long our currently available cash, cash equivalents and investments will be sufficient to finance our operations and business plans and statements about our future capital needs;
- our current and planned investment in and activities in preparation for a potential commercial launch of eteplirsen, including negotiating and entering into commercial and supply contracts, scaling up manufacturing and hiring for pre-launch and commercial positions and the impact of winding down or terminating these commitments if the FDA does not approve our eteplirsen NDA;
- our ability to raise additional funds to support our business plans and the impact of our credit and security agreement with MidCap Financial on our financial condition and future operations;
- our expectations regarding our ability to become a leading developer and marketer of PMO-based and RNA-targeted therapeutics and commercial viability of our product candidates, chemistries and technologies;
- the potential safety, efficacy, potency and utility of our product candidates, chemistries and technologies in the treatment of Duchenne muscular dystrophy ("DMD") and in rare, infectious and other diseases;
    - our expectations regarding the timing, completion and receipt of results from our ongoing development programs for our pipeline of product candidates including their potential consistency with prior results;
- our ability to effectively manage the clinical trial process for our product candidates on a timely basis, including our ability to conduct a placebo-controlled confirmatory study for eteplirsen in the U.S. using an exon 53 skipping product candidate that depends on our ability to satisfactorily and timely respond to FDA requests with respect to preclinical data on the exon 53 skipping product candidate;
- our expectations regarding our ability to engage a number of manufacturers with sufficient capability and capacity to meet our manufacturing needs, including with respect to the manufacture of subunits, drug substance ("API's") and drug product, within the time frames and quantities needed to provide our product candidates, including eteplirsen, to patients in larger scale clinical trials or in potential commercial quantities, and meet regulatory and Company quality control requirements;
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the impact of regulations as well as regulatory decisions by the FDA and other regulatory agencies on our business, including with respect to our eteplirsen NDA submission and any issuance of an Emergency Use Authorization ("EUA") for our product candidates intended to treat Ebola virus and Marburg virus, as well as the development of our product candidates and our financial and contractual obligations;

- our expectations regarding the potential markets for our product candidates;

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

All forward-looking statements are based on information available to us on the date of this Quarterly Report on Form 10-Q and we will not update any of the forward-looking statements after the date of this Quarterly Report on Form 10-Q, except as required by law or the rules and regulations of the U.S. Securities and Exchange Commission ("SEC"). We caution readers not to place undue reliance on forward-looking statements. Our actual results could differ materially from those discussed in this Quarterly Report on Form 10-Q. The forward-looking statements contained in this Quarterly Report on Form 10-Q, and other written and oral forward-looking statements made by us from time to time, are subject to certain risks and uncertainties that could cause actual results to differ materially from those

anticipated in the forward-looking statements. Applicable risks and uncertainties include, among others, the fact that: the FDA may not approve eteplirsen as a DMD therapeutic; we may be delayed or may not be able to comply with the FDA's requests for additional information in connection with our eteplirsen NDA; the additional information and data we collect for

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

Overview

We are a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying DMD drug candidates, including our lead DMD product candidate, eteplirsen, designed to skip exon 51. On August 25, 2015, we announced the FDA's filing of our NDA for eteplirsen for the treatment of DMD amenable to exon 51 skipping. Eteplirsen is under priority review with a PDUFA action date of February 26, 2016. We are also developing therapeutics using our technology for the treatment of drug resistant bacteria and infectious, rare and other human diseases.

Our RNA-targeted technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. Currently, there are no approved disease-modifying therapies for DMD in the U.S.; the FDA, however, has accepted NDAs for eteplirsen, our lead product candidate for DMD, and for drisapersen, which is being developed by BioMarin Pharmaceuticals, Inc., each for patients amenable to exon 51 skipping. Approximately 13 percent of people with DMD are estimated to have the

mutation amenable to exon 51 skipping. If we are successful in our development efforts, eteplirsen will address a severe and currently unmet medical need. We are in the process of conducting or initiating several studies for product candidates designed to skip exons 45, 51 and 53 in the U.S. and the European Union (the "E.U."). These comprise (i) studies to further evaluate eteplirsen that include an open label extension of our Phase IIb eteplirsen study, a confirmatory trial in ambulatory patients, a study on participants with advanced stage DMD and a study with participants with early stage DMD, (ii) a dose-ranging study for our product candidate designed to skip exon 45, (iii) a 2-part randomized, double blind, placebo-controlled, dose titration safety, tolerability and pharmacokinetics study (Part I) followed by an open label efficacy and safety study (Part II) with a product candidate designed to skip exon 53 and (iv) a placebo-controlled confirmatory study with product candidates designed to skip exons 45 and 53. Our discovery and research programs include collaborations with various third parties and focus on developing therapeutics in rare, genetic, anti-infective, neuromuscular and central nervous system diseases among other diseases. We are exploring the application of our proprietary PMO platform technology in various diseases including drug resistant bacteria, DMD, Becker muscular dystrophy, progeria, adult onset Pompe disease and lupus.

The basis of our novel RNA-targeted therapeutics is the PMO chemistries. Our next generation PMO-based chemistries include PMO-X®, PMOplus® and PPMO. PMOs are highly resistant to degradation by enzymes, potentially enabling robust and sustained

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

We believe we have developed proprietary state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of our product candidates to support clinical development as well as potential commercialization. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support production of certain of our product candidates and their components. We currently do not have any of our own internal mid-to-large scale manufacturing capabilities to support our product candidates.

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

On October 9, 2015, we completed a public offering whereby we sold 3,250,000 shares of common stock at a price of $39.00 per share. In addition, we have granted the underwriters a 30-day option to purchase an additional 487,500 shares of common stock at a price of $39.00 per share. There can be no assurance that the underwriters will exercise the option. We received aggregate net proceeds from the offering of approximately $120.0 million, after deducting the underwriting discounts and offering-related transaction costs.

As of September 30, 2015, we had approximately $111.4 million of cash, cash equivalents and investments, consisting of $55.8 million of cash and cash equivalents, $44.1 million of short-term investments and $11.5 million of restricted cash and investments. We believe that the balance of our cash, cash equivalents and investments as of September 30, 2015, together with the net proceeds of approximately $120.0 million received from our common stock offering completed on October 9, 2015, is sufficient to fund our current operational plan for the next twelve months. We may pursue additional cash resources through public or private financings, establish credit or debt facilities or collaborate with, or license our technology to, other companies.

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

Key Financial Metrics

Revenue

Revenue from Research Contracts and Other Grants. In the periods presented in this report, substantially all of our revenue was derived from our former research and development contracts with and grants from the U.S. government. As of December 31, 2014, we had completed all development activities under our contracts with the U.S. government. We recognize revenue from U.S. government research contracts and grants during the period in which the related expenses are incurred and present such revenue and related expenses on a gross basis in the unaudited condensed

consolidated financial statements. Our government contracts are subject to government audits, which may result in catch-up adjustments.

If a technology, right, product or service is separate and independent of our performance under other elements of an arrangement, we defer recognition of non-refundable up-front fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, such up-front fees are deferred and recognized over the period of continuing involvement. As of September 30, 2015, we had deferred revenue of $3.3 million, which represents up-front fees which we may recognize as revenue upon settlement of certain obligations.

Expenses

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

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

Future research and development expenses may increase as our internal projects, such as those for our DMD product candidates, enter or proceed through later stage clinical development. We are currently conducting various clinical trials for eteplirsen, including a confirmatory trial in the U.S. We completed Part I and have started conducting Part II of a Phase I/IIa clinical trial for an exon 53 skipping product candidate in the E.U. and have initiated a dose-ranging study for our exon 45 skipping product candidate in the U.S. We also plan to include our exon 45 and exon 53 skipping product candidates in our planned placebo-controlled confirmatory clinical trial in the U.S. and the E.U. The remainder of our research and development programs are in various stages of research and pre-clinical development. However, our research and development efforts may not result in any approved products. Product candidates that appear promising at early stages of development may not reach the market for a variety of reasons. Similarly, any of our product candidates may be found to be unsafe or ineffective during clinical trials, may have clinical trials that take longer to complete than anticipated, may fail to receive necessary regulatory approvals, or may prove impracticable to manufacture in commercial quantities at reasonable cost and with acceptable quality.

As a result of these uncertainties and the other risks inherent in the drug development process, we cannot determine the duration or completion costs of current or future clinical stages of any of our product candidates. Similarly, we cannot determine when, if, or to what extent we may generate revenue from the commercialization of any product candidate. The time frame for development of any product candidate, associated development costs and the probability of regulatory and commercial success vary widely.

General and Administrative. General and administrative expenses consist principally of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, human resource and other general and administrative functions. Other general and administrative expenses include an allocation of our facility costs and professional fees for legal, consulting and accounting services.

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

Gain (Loss) on Change in Warrant Valuation. Warrants issued in connection with our January and August 2009 financings were classified as liabilities as opposed to equity due to their settlement terms. These warrants were non-cash liabilities and we were not required to expend any cash to settle these liabilities. The fair value of these warrants was recorded on our unaudited condensed consolidated balance sheets at the date of issuance. The warrants were marked to market at each financial reporting period, with changes in the fair value recorded as “Gain (loss) on change in warrant valuation” in our unaudited condensed consolidated statements of operations and comprehensive

loss. The fair value of the warrants was determined using the Black-Scholes-Merton option-pricing model, which required the use of significant judgment and estimates related to the inputs used in the model. All warrants issued in January and August 2009 were exercised or expired during 2014.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations is based upon our unaudited condensed consolidated financial statements included elsewhere in this report. The preparation of our unaudited condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States ("U.S. GAAP") requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and related disclosure of contingent assets and liabilities for the periods presented. Some of these judgments can be subjective and complex and, consequently, actual results may differ from these estimates. For any given individual estimate or assumption we make, there may also be other estimates or assumptions that are reasonable. We believe that the estimates and judgments upon which we rely are reasonable based upon historical experience and information available to us at the time when we make these estimates and judgments. To the extent

there are material differences between these estimates and actual results, our unaudited condensed consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates.

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

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

There have been no material changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2014 filed with the SEC on February 26, 2015.

Results of Operations for the Three and Nine Months Ended September 30, 2015 and 2014

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

	For the Three Months Ended September 30, 2015	2014	Change	Change
	(in thousands, except per share amounts)		$	%
Revenue from research contracts and other grants	$—	$ 1,059	$(1,059	