

BIOMARIN PHARMACEUTICAL INC

Form 10-Q

November 02, 2015

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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 September 30, 2015

Or

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_.

Commission File Number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware 68-0397820  
(State or other jurisdiction of (I.R.S. Employer

incorporation or organization) Identification No.)

770 Lindero Street, San Rafael, California 94901  
(Address of principal executive offices) (Zip Code)

(415) 506-6700

(Registrant's telephone number including area code)

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.

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

Applicable only to issuers involved in bankruptcy proceedings during the preceding five years:

Indicate by check mark whether the registrant has filed all documents and reports required to be filed by Sections 12, 13 or 15(d) of the Securities Exchange Act of 1934 subsequent to the distribution of securities under a plan confirmed by a court. Yes  No

Applicable only to corporate issuers:

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 161,251,715 shares of common stock, par value \$0.001, outstanding as of October 23, 2015.

BIOMARIN PHARMACEUTICAL INC.

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## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED BALANCE SHEETS

September 30, 2015 and December 31, 2014

(In thousands of U.S. dollars, except share amounts)

	September 30, 2015	December 31, 2014 <sup>(1)</sup>
<b>ASSETS</b>	(unaudited)	
Current assets:		
Cash and cash equivalents	\$376,346	\$875,486
Short-term investments	241,984	69,706
Accounts receivable, net (allowance for doubtful accounts: \$80 and \$490, at September 30, 2015 and December 31, 2014, respectively)	148,949	144,472
Inventory	262,100	199,452
Current deferred tax assets	30,880	31,203
Other current assets	115,330	111,835
<b>Total current assets</b>	<b>1,175,589</b>	<b>1,432,154</b>
Noncurrent assets:		
Long-term investments	514,381	97,856
Property, plant and equipment, net	604,513	523,516
Intangible assets, net	920,943	156,578
Goodwill	202,392	54,258
Long-term deferred tax assets	146,245	159,771
Other assets	60,790	66,320
<b>Total assets</b>	<b>\$3,624,853</b>	<b>\$2,490,453</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$296,200	\$231,844
Short-term contingent acquisition consideration payable	94,291	3,895
<b>Total current liabilities</b>	<b>390,491</b>	<b>235,739</b>
Noncurrent liabilities:		
Long-term convertible debt	667,793	657,976
Long-term contingent acquisition consideration payable	34,874	38,767
Long-term deferred tax liabilities	193,202	—
Other long-term liabilities	45,853	30,077
<b>Total liabilities</b>	<b>1,332,213</b>	<b>962,559</b>
Stockholders' equity:		
Common stock, \$0.001 par value: 250,000,000 shares authorized at September 30, 2015 and December 31, 2014: 161,226,410 and 149,093,647 shares issued and outstanding at September 30, 2015 and December 31, 2014, respectively	162	149
Additional paid-in capital	3,371,377	2,359,744
Company common stock held by Nonqualified Deferred Compensation Plan	(13,897 )	(9,695 )

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Accumulated other comprehensive income	25,184	27,466
Accumulated deficit	(1,090,186)	(849,770 )
Total stockholders' equity	2,292,640	1,527,894
Total liabilities and stockholders' equity	\$3,624,853	\$2,490,453

(1) December 31, 2014 balances were derived from the audited Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014, filed with the Securities and Exchange Commission (the SEC) on March 2, 2015.

The accompanying notes are an integral part of these Condensed Consolidated Financial Statements.

## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)

Three and Nine Months Ended September 30, 2015 and 2014

(In thousands of U.S. dollars, except per share amounts)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
<b>REVENUES:</b>				
Net product revenues	\$207,767	\$173,416	\$658,102	\$510,664
Collaborative agreement revenues	131	353	849	1,274
Royalty, license and other revenues	1,006	2,780	3,008	7,278
Total revenues	208,904	176,549	661,959	519,216
<b>OPERATING EXPENSES:</b>				
Cost of sales	36,719	29,920	109,410	83,946
Research and development	158,713	125,686	458,688	319,476
Selling, general and administrative	94,044	74,604	288,364	202,388
Intangible asset amortization and contingent consideration	1,301	2,636	17,518	15,041
Gain on sale of intangible asset	—	(67,500)	—	(67,500)
Total operating expenses	290,777	165,346	873,980	553,351
<b>INCOME (LOSS) FROM OPERATIONS</b>	<b>(81,873)</b>	<b>11,203</b>	<b>(212,021)</b>	<b>(34,135)</b>
Equity in the loss of BioMarin/Genzyme LLC	(186)	(225)	(539)	(1,102)
Interest income	1,344	1,435	3,050	4,293
Interest expense	(9,447)	(9,118)	(28,911)	(27,445)
Debt conversion expense	—	—	(163)	(674)
Other expense	(281)	(74)	(9,105)	(68)
<b>INCOME (LOSS) BEFORE INCOME TAXES</b>	<b>(90,443)</b>	<b>3,221</b>	<b>(247,689)</b>	<b>(59,131)</b>
Provision for (benefit from) income taxes	483	(4,224)	(7,273)	5,041
<b>NET INCOME (LOSS)</b>	<b>\$(90,926)</b>	<b>\$7,445</b>	<b>\$(240,416)</b>	<b>\$(64,172)</b>
<b>NET INCOME (LOSS) PER SHARE, BASIC</b>	<b>\$(0.57)</b>	<b>\$0.05</b>	<b>\$(1.51)</b>	<b>\$(0.44)</b>
<b>NET INCOME (LOSS) PER SHARE, DILUTED</b>	<b>\$(0.60)</b>	<b>\$0.05</b>	<b>\$(1.51)</b>	<b>\$(0.44)</b>
Weighted average common shares outstanding, basic	160,886	147,016	159,647	145,724
Weighted average common shares outstanding, diluted	161,134	159,304	159,647	145,724
<b>COMPREHENSIVE INCOME (LOSS)</b>				
	<b>\$(98,203)</b>	<b>\$16,693</b>	<b>\$(242,698)</b>	<b>\$(50,001)</b>

The accompanying notes are an integral part of these Condensed Consolidated Financial Statements.

## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Nine Months Ended September 30, 2015 and 2014

(In thousands of U.S. dollars)

(Unaudited)

	2015	2014
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>		
Net loss	\$(240,416 )	\$(64,172 )
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	45,306	39,905
Non-cash interest expense	21,243	20,305
Accretion of discount on investments	1,616	5,748
Stock-based compensation	84,465	55,251
Gain on sale of intangible asset	—	(67,500 )
Gain on termination of lease	—	(8,893 )
Gain on sale of equity investment	(3,022 )	—
Impairment of assets	12,802	—
Deferred income taxes	14,629	(12,373 )
Excess tax benefit from stock option exercises	(463 )	(205 )
Unrealized foreign exchange (gain) loss on forward contracts	(16,491 )	2,354
Non-cash changes in the fair value of contingent acquisition consideration payable	15,101	11,202
Other	1,059	2,136
Changes in operating assets and liabilities:		
Accounts receivable, net	146	(3,482 )
Inventory	(61,980 )	(41,114 )
Other current assets	(27,970 )	(9,783 )
Other assets	(1,391 )	(6,708 )
Accounts payable and accrued liabilities	924	30,930
Other long-term liabilities	790	(116 )
Net cash used in operating activities	(153,652 )	(46,515 )
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>		
Purchases of property, plant and equipment	(123,844 )	(89,295 )
Funds held in escrow for the purchase of real property	(12,500 )	—
Maturities and sales of investments	261,786	207,476
Purchase of available-for-sale investments	(842,873 )	(448,938 )
Proceeds from sale of intangible asset	—	67,500
Purchase of promissory note	(3,326 )	—
Business acquisitions, net of cash acquired	(538,392 )	—
Other	—	(3,100 )
Net cash used in investing activities	(1,259,149 )	(266,357 )
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>		
Proceeds from exercises of stock options and Employee Stock Purchase Plan (the ESPP)	51,515	37,635
Taxes paid related to net share settlement of equity awards	(21,968 )	(7,246 )
Proceeds from public offering of common stock, net	888,257	117,464

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Excess tax benefit from stock option exercises	463	205
Payment of contingent acquisition consideration payable	—	(4,691 )
Other	(2,062 )	(691 )
Net cash provided by financing activities	916,205	142,676
Effect of exchange rate changes on cash	(2,544 )	(580 )
NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	(499,140 )	(170,776)
Cash and cash equivalents:		
Beginning of period	\$875,486	\$568,781
End of period	\$376,346	\$398,005
SUPPLEMENTAL CASH FLOW DISCLOSURES:		
Cash paid for interest, net of interest capitalized into fixed assets	4,979	4,759
Cash paid for income taxes	15,377	22,378
Stock-based compensation capitalized into inventory	8,271	5,663
Depreciation capitalized into inventory	11,005	7,989
SUPPLEMENTAL CASH FLOW DISCLOSURES FROM INVESTING AND FINANCING		
ACTIVITIES:		
Decrease in accounts payable and accrued liabilities related to fixed assets	(11,386 )	(2,762 )
Conversion of convertible debt	8,957	16,482
Release of escrow balance for purchase of San Rafael Corporate Center	—	116,500

The accompanying notes are an integral part of these Condensed Consolidated Financial Statements.



BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(1) NATURE OF OPERATIONS AND BUSINESS RISKS

BioMarin Pharmaceutical Inc. (the Company or BioMarin), a Delaware corporation, develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. BioMarin selects product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. The Company's product portfolio is comprised of five approved products and multiple clinical and pre-clinical product candidates. The Company's approved products are Vimizim (elosulfase alfa), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

The Company expects to continue to finance future cash needs that exceed its operating activities primarily through its current cash, cash equivalents, short-term and long-term investments, and to the extent necessary, through proceeds from equity or debt financings, loans and collaborative agreements with corporate partners. If the Company elects to increase its spending on development programs significantly above current long-term plans or enters into potential licenses and other acquisitions of complementary technologies, products or companies, the Company may need additional capital.

The Company is subject to a number of risks, including: the financial performance of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse; the potential need for additional financings; the Company's ability to successfully commercialize its approved product candidates; the uncertainty of the Company's research and development (R&D) efforts resulting in future successful commercial products; the Company's ability to successfully obtain regulatory approval for new products; significant competition from larger organizations; reliance on the proprietary technology of others; dependence on key personnel; uncertain patent protection; dependence on corporate partners and collaborators; and possible restrictions on reimbursement from governmental agencies and healthcare organizations, as well as other changes in the health care industry.

(2) BASIS OF PRESENTATION

The accompanying Condensed Consolidated Financial Statements have been prepared pursuant to the rules and regulations of the SEC for Quarterly Reports on Form 10-Q and do not include all of the information and note disclosures required by U.S. generally accepted accounting principles (U.S. GAAP) for complete financial statements, although the Company believes that the disclosures herein are adequate to ensure that the information presented is not misleading. The Condensed Consolidated Financial Statements should therefore be read in conjunction with the Consolidated Financial Statements and Notes thereto for the fiscal year ended December 31, 2014 included in the Company's Annual Report on Form 10-K.

The accompanying Condensed Consolidated Financial Statements have been prepared in accordance with U.S. GAAP, which requires management to make estimates and assumptions that affect amounts reported in the Condensed Consolidated Financial Statements and accompanying disclosures. Although these estimates are based on management's best knowledge of current events and actions that the Company may undertake in the future, actual results may be different from those estimates. The Condensed Consolidated Financial Statements reflect all

adjustments of a normal, recurring nature that are, in the opinion of management, necessary for a fair presentation of results for these interim periods. The results of operations for the three and nine months ended September 30, 2015 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2015.

The Company has evaluated events and transactions subsequent to the balance sheet date. Based on this evaluation, the Company is not aware of any events or transactions that occurred subsequent to the balance sheet date but prior to filing this Quarterly Report on Form 10-Q that would require recognition or disclosure in the Condensed Consolidated Financial Statements, except for the transactions disclosed in Note 21.

### (3) SIGNIFICANT ACCOUNTING POLICIES

There have been no material changes to the Company's significant accounting policies during the nine months ended September 30, 2015, as compared to the significant accounting policies disclosed in Note 3 of the Consolidated Financial Statements in the Company's Annual Report on Form 10-K for the year ended December 31, 2014.

#### Reclassifications

Certain items in the Company's prior year Condensed Consolidated Financial Statements have been reclassified to conform to the current presentation.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

#### (4) RECENT ACCOUNTING PRONOUNCEMENTS

Except as described below, there have been no new accounting pronouncements or changes to accounting pronouncements during the nine months ended September 30, 2015, as compared to the recent accounting pronouncements described in Note 4 of the Company's Annual Report on Form 10-K for the year-ended December 31, 2014, that are of significance or potential significance to the Company.

In April 2015, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. ASU 2015-03, Interest–Imputation of Interest (Subtopic 835-30), which changes the presentation of debt issuance costs in financial statements. ASU 2015-03 requires an entity to present such costs in the balance sheet as a direct deduction from the related debt liability rather than as an asset. Amortization of the costs will continue to be reported as interest expense. It is effective for annual reporting periods beginning after December 15, 2016, which for the Company is January 1, 2017. Early adoption is permitted. The new guidance will be applied retrospectively to each prior period presented. The Company does not expect that the adoption of this standard will have a material impact on the Company's results of operations nor will it result in a reclassification of the balance of deferred offering costs at the time of adoption. If the standard were adopted as of September 30, 2015, the reclassification would approximate \$12.6 million.

In July 2015, the FASB deferred the effective date for ASU No. 2014-09 (ASU 2014-09), Revenue from Contracts with Customers, by one year. ASU 2014-09 will supersede the revenue recognition requirements in Revenue Recognition (Topic 605) and requires entities to recognize revenue in a way that depicts the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. ASU 2014-09 is effective for annual reporting periods beginning after December 15, 2017, including interim periods within that reporting period, which for the Company is January 1, 2018. Early adoption is not permitted. The new standard can be applied retrospectively to each prior reporting period presented or retrospectively with the cumulative effect of the change recognized at the date of the initial application in retained earnings. The Company is currently evaluating the potential impact the adoption of ASU 2014-09 will have on its consolidated financial statements and has not yet selected a transition method.

In July 2015, the FASB issued ASU No. 2015-11, Inventory (ASU 2015-11), which requires an entity to measure inventory within the scope at the lower of cost and net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The effective date for the standard is for fiscal years beginning after December 15, 2016, which for the Company is January 1, 2017. Early adoption is permitted. The new standard is to be applied prospectively. The Company does not expect ASU 2015-11 to have a material impact on its consolidated financial statements.

In September 2015, the FASB issued ASU No. 2015-16, Simplifying the Accounting for Measurement-Period Adjustments (ASU 2015-16). The amended guidance requires that an acquirer recognize adjustments to provisional amounts that are identified during the measurement period in the reporting period in which the adjustment amounts

are determined. The amendments are effective prospectively for the fiscal years, and the interim reporting periods within those years, beginning on or after December 15, 2015 and early adoption is permitted. The Company is currently evaluating the potential impact the adoption of ASU 2015-16 will have on its consolidated financial statements and has not elected to early adopt ASU 2015-16.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

## (5) ACQUISITIONS

## Prosensa Holding N.V.

On January 29, 2015, the Company completed the acquisition of Prosensa Holding N.V. (Prosensa), a public limited liability company organized under the laws of the Netherlands, for a total purchase price of \$751.5 million. In connection with the acquisition of Prosensa, the Company recognized transaction costs of \$9.7 million, of which \$2.7 million and \$7.0 million, respectively, was recognized in the year ended December 31, 2014 and the nine months ended September 30, 2015.

Prosensa was an innovative biotechnology company engaged in the discovery and development of ribonucleic acid (RNA)-modulating therapeutics for the treatment of genetic disorders. Prosensa's primary focus was on rare neuromuscular and neurodegenerative disorders with a large unmet medical need, including subsets of patients with Duchenne muscular dystrophy (DMD), myotonic dystrophy and Huntington's disease. Prosensa's clinical portfolio of RNA-based product candidates was focused on the treatment of DMD. Each of Prosensa's DMD compounds has been granted orphan drug status in the United States (the U.S.) and the European Union (the EU). Prosensa's lead product, Kyndrisa formerly referred to as drisapersen, is currently under review as part of a rolling new drug application (NDA) with the Food and Drug Administration (the FDA). On April 27, 2015, the Company announced the completion of the rolling submission of the NDA to the FDA. On June 8, 2015, the Company announced the submission of a marketing authorization application (MAA) for Kyndrisa with the European Medicines Agency (the EMA).

In connection with its acquisition of Prosensa, the Company made cash payments totaling \$680.1 million, which were comprised of \$620.7 million for approximately 96.8% of Prosensa's ordinary shares (the Prosensa Shares), \$38.6 million for the options that vested pursuant to the Company's tender offer for the Prosensa Shares and \$20.8 million to the remaining Prosensa shareholders that did not tender their shares under the tender offer. Additionally, for each Prosensa Share, the Company issued one non-transferable contingent value right (the CVR), which represents the contractual right to receive a cash payment of up to \$4.14 per Prosensa Share, or an aggregate of approximately \$160.0 million (undiscounted), upon the achievement of certain product approval milestones. The fair value of the CVRs and acquired in-process research and development (IPR&D) on the acquisition date was \$71.4 million and \$772.8 million, respectively. The acquisition date fair value of the CVRs and IPR&D was estimated by applying a probability-based income approach utilizing an appropriate discount rate. Key assumptions include a discount rate and various probability factors. See Note 15 to these Condensed Consolidated Financial Statements for additional discussion regarding fair value measurements of the CVRs, which is included in contingent acquisition consideration payable.

The following table presents the allocation of the purchase consideration for the Prosensa acquisition, including the CVRs, based on fair value.

Cash and cash equivalents	\$ 141,669
Trade accounts receivable	3,086

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Other current assets	1,537
Property, plant and equipment	2,683
Intangible assets	497
Other assets	104
Acquired IPR&D	772,808
Total identifiable assets acquired	922,384
Accounts payable and accrued expenses	(68,799 )
Debt assumed	(57,053 )
Deferred tax liability	(193,202)
Total liabilities assumed	(319,054)
Net identifiable assets acquired	603,330
Goodwill	148,134
Net assets acquired	\$751,464

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

A substantial portion of the assets acquired consisted of IPR&D related to Prosensa's product candidates Kyndrisa and exons PRO 044 and PRO 045, which are considered to be indefinite-lived assets until completion or abandonment of the associated research and development (R&D) efforts. The Company determined that the estimated acquisition-date fair value of the intangible assets related to Kyndrisa and Prosensa's other primary product candidates, PRO 044 and PRO 045, was \$731.8 million, \$16.9 million and \$24.1 million, respectively.

The deferred tax liability relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, which are not deductible for tax purposes.

Prosensa's results of operations prior to and since the acquisition date are insignificant to the Company's Condensed Consolidated Financial Statements.

See Note 10 to these Condensed Consolidated Financial Statements for further discussion of the indefinite-lived intangible assets.

## San Rafael Corporate Center

In March 2014, the Company completed the acquisition of the real estate commonly known as the San Rafael Corporate Center (SRCC), located in San Rafael, California. SRCC is a multi-building, commercial property where, prior to the acquisition, the Company was leasing a certain portion of the space for its headquarters and related operating activities. The purpose of this acquisition is to allow for future expansion of the Company's corporate headquarters to accommodate anticipated headcount growth. The acquisition of SRCC has been accounted for as a business combination because the building and the in-place leases met the definition of a business in Accounting Standards Codification 805 (ASC 805), Business Combinations. The fair value of the consideration paid for SRCC was \$116.5 million, all of which was paid in cash.

The following table summarizes the estimated fair values of assets acquired as of the date of acquisition:

	Estimated	Estimated
	Fair Value	Useful Lives
Building and improvements	\$ 94,414	50 years
Land	14,565	
Land improvements	3,616	10 years
		Remaining
Lease intangible assets	3,905	lease terms
Total identifiable net assets	\$ 116,500	

The fair values assigned to tangible and identifiable intangible assets acquired are based on management's estimates and assumptions using the information that was available as of the date of the acquisition. The Company believes that the information provides a reasonable basis for estimating the fair values of assets acquired.

The following table sets forth the fair value of the components of the identifiable lease intangible assets acquired by asset class as of the date of acquisition:

Above market leases	\$351
In-place leases	3,554
Total lease intangible assets subject to amortization	\$3,905

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The value of any lease intangible assets (such as in-place and above-market leases) is estimated to be equal to the property owners' avoidance of costs necessary to release the property for a lease term equal to the remaining primary in-place lease term and the value of investment-grade tenancy, which is derived by estimating, based on a review of the market, the cost to be borne by a property owner to replicate a market lease for the remaining in-place term. These costs consist of: (i) rent lost during downtime (e.g., assumed periods of vacancy), (ii) estimated expenses that would be incurred by the property owner during periods of vacancy, (iii) rent concessions (e.g., free rent), (iv) leasing commissions and (v) tenant improvement allowances. The Company determined these values using management's estimates along with third-party appraisals. The Company will amortize the capitalized value of lease intangible assets over the remaining lives of the underlying leases. Lease intangible assets are amortized as a reduction in (addition to) third-party tenant revenue, which is included in Royalty, License and Other Revenues on the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2015 and 2014.

The amount of third-party tenant revenue included in the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2015 was \$0.6 million and \$2.1 million, respectively, compared to \$1.3 million and \$3.1 million for the three and nine months ended September 30, 2014, respectively. Amortization expense for each of the three and nine months ended September 30, 2015 and 2014 were \$0.3 million and \$1.0 million, respectively. The amount of net income/loss from third-party tenants for each of the three and nine months ended September 30, 2015 and 2014 was insignificant to the Company's Condensed Consolidated Statement of Comprehensive Income (Loss).

SRCC's results of operations prior to the acquisition were insignificant to the Company's Condensed Consolidated Financial Statements.

Included in Selling, General and Administrative (SG&A) expenses during the nine months ended September 30, 2014 are transaction costs incurred in connection with the acquisition of SRCC of \$0.3 million. The Company recognized a gain of \$8.8 million in the nine months ended September 30, 2014 due to the early termination of the Company's pre-existing lease and the realization of the remaining balance in deferred rent and the reversal of the related asset retirement obligation upon acquisition of SRCC. \$2.7 million and \$6.1 million of the gain were included in SG&A and R&D expenses, respectively, which is consistent with the Company's allocation practices for facility costs for this previously leased space.

## (6) STOCKHOLDERS' EQUITY

In January 2015, the Company sold 9,775,000 shares of its common stock at a price of \$93.25 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. The Company received net proceeds of approximately \$888.3 million from this public offering after underwriter's discount

and offering costs.

(7) NET INCOME (LOSS) PER COMMON SHARE

Potentially issuable shares of common stock include shares issuable upon the exercise of outstanding employee stock option awards, common stock issuable under the Company's Amended and Restated 2006 Employee Stock Purchase Plan (the ESPP), unvested restricted stock units (RSUs), common stock held by the Company's Nonqualified Deferred Compensation Plan (the NQDC) and contingent issuances of common stock related to convertible debt.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The following table sets forth the computation of basic and diluted earnings per common share (in thousands of common shares):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
<b>Numerator:</b>				
Net income (loss), basic	\$(90,926 )	\$7,445	\$(240,416)	\$(64,172 )
Interest expense related to the 2017 Notes	—	156	—	—
Gain on common stock held by the NQDC	(4,980 )	—	—	—
Net income (loss), diluted	\$(95,906 )	\$7,601	\$(240,416)	\$(64,172 )
<b>Denominator:</b>				
Weighted-average common shares outstanding, basic	160,886	147,016	159,647	145,724
<b>Effect of dilutive securities:</b>				
Options to purchase common stock	—	9,275	—	—
Common stock issuable under the 2017 Notes	—	2,238	—	—
Unvested RSUs	—	642	—	—
Potentially issuable common stock of ESPP purchases	—	133	—	—
Common shares held by the NQDC	248	—	—	—
Weighted-average common shares outstanding, diluted	161,134	159,304	159,647	145,724
Net income (loss) per common share, basic	\$(0.57 )	\$0.05	\$(1.51 )	\$(0.44 )
Net income (loss) per common share, diluted	\$(0.60 )	\$0.05	\$(1.51 )	\$(0.44 )

In addition to the equity instruments included in the table above, the table below presents potential shares of common stock that were excluded from the computation as they were anti-dilutive using the treasury stock method (in thousands of common shares):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Options to purchase common stock	10,503	3,491	10,503	12,780
Common stock issuable under the 2017 Notes	1,553	—	1,553	2,238
Common stock issuable under the 2018 and 2020 Notes	7,966	7,966	7,966	7,966
Unvested restricted stock units	1,757	559	1,633	1,216
Potentially issuable common stock for ESPP purchases	229	—	220	135
Common stock held by the NQDC	—	224	248	224
Total number of potentially issuable shares	22,008	12,240	22,123	24,559

The effect of the Company's 0.75% senior subordinated convertible notes due in 2018 (the 2018 Notes) and the Company's 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes, and together with the 2018 Notes, the Notes) was excluded from the diluted net loss per common share since they may be settled in cash or shares at the Company's option and the Company's current intention is to settle up to the principal amount of the converted notes in cash and any excess conversion value (conversion spread) in shares of the Company's common stock. As a result, during the three and nine months ended September 30, 2014 the 2018 Notes and the 2020 Notes had no effect on diluted net loss per share as the Company's stock price did not exceed the conversion price of \$94.15 per share for the Notes. Although the Company's stock price exceeded the conversion price at September 30, 2015, the potential shares issuable under the Notes were excluded from the calculation of diluted loss per share as they were anti-dilutive using the if-converted method.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

## (8) INVESTMENTS

All investments were classified as available-for-sale at September 30, 2015 and December 31, 2014. The amortized cost, gross unrealized holding gains or losses, and fair value of the Company's available-for-sale securities by major security type at September 30, 2015 and December 31, 2014 are summarized in the tables below:

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at September 30, 2015
Certificates of deposit	\$ 57,687	\$ 1	\$ —	\$ 57,688
Corporate debt securities	413,955	216	(757 )	413,414
Commercial paper	60,892	—	—	60,892
U.S. government agency securities	223,963	278	—	224,241
Greek government-issued bonds	50	80	—	130
Total	\$ 756,547	\$ 575	\$ (757 )	\$ 756,365

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at December 31, 2014
Certificates of deposit	\$ 72,302	\$ 1	\$ —	\$ 72,303
Corporate debt securities	95,478	—	(342 )	95,136
Greek government-issued bonds	50	73	—	123
Total	\$ 167,830	\$ 74	\$ (342 )	\$ 167,562

The Company has two investments in marketable equity securities measured using quoted prices in their respective active markets that are collectively considered strategic investments. As of September 30, 2015, the fair value of the Company's marketable equity securities was \$21.4 million, which included an unrealized gain of \$16.0 million. As of December 31, 2014, the fair value of the Company's marketable equity securities was \$30.8 million, which included an unrealized gain of \$18.3 million. These investments are recorded in Other Assets in the Company's Condensed Consolidated Balance Sheets.

The fair values of available-for-sale securities by contractual maturity were as follows:

	September 30, 2015	December 31, 2014
Maturing in one year or less	\$ 241,984	\$ 69,706
Maturing after one year through five years	514,381	97,856
Total	\$ 756,365	\$ 167,562

Impairment assessments are made at the individual security level each reporting period. When the fair value of an investment is less than its cost at the balance sheet date, a determination is made as to whether the impairment is other-than-temporary and, if it is other-than-temporary, an impairment loss is recognized in earnings equal to the difference between the investment's amortized cost and fair value at such date. As of September 30, 2015, some of the Company's investments were in an unrealized loss position. However, the Company has the ability and intent to hold all investments that have been in a continuous loss position until maturity or recovery, thus no other-than-temporary impairment is deemed to have occurred.

See Note 15 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of the Company's available-for-sale securities.

#### (9) GOODWILL

Goodwill is tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in the circumstances that would indicate a reduction in the fair value of the goodwill below its carrying amount.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The following table represents the changes in goodwill for the nine months ended September 30, 2015:

Balance at December 31, 2014	\$54,258
Addition of goodwill related to the acquisition of Prosensa	148,134
Balance at September 30, 2015	\$202,392

## (10) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	September 30, 2015	December 31, 2014
<b>Intangible assets:</b>		
Finite-lived intangible assets	\$ 123,732	\$ 123,365
Indefinite-lived intangible assets	812,088	74,430
Indefinite-lived intangible assets held-for-sale	35,150	—
<b>Gross intangible assets:</b>	<b>970,970</b>	<b>197,795</b>
Less: Accumulated amortization	(50,027 )	(41,217 )
<b>Net carrying value</b>	<b>\$ 920,943</b>	<b>\$ 156,578</b>

## Indefinite-Lived Intangible Assets

IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated R&D efforts. During the period the assets are considered indefinite-lived, they will not be amortized but will be tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in circumstances that would indicate a reduction in the fair value of the IPR&D assets below their respective carrying amounts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point in time.

In August 2015, the Company announced it had entered into an asset purchase agreement under (the Asset Purchase Agreement) which Medivation Inc. (Medivation), would acquire worldwide rights to talazoparib. As of September 30, 2015, the carrying value of the talazoparib intangible assets were classified as held-for-sale and totaled \$35.2 million, which was included in Intangible Assets, net on the Company's Condensed Consolidated Balance Sheet. The Company completed the sale of talazoparib to Medivation on October 6, 2015. See Note 21 to these Condensed Consolidated Financial Statements for additional discussion.

See Note 6 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014 for additional information related to the Company's Intangible Assets.



## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

## (11) PROPERTY, PLANT AND EQUIPMENT

Property, plant and equipment, net consisted of the following:

	September 30, 2015	December 31, 2014
Leasehold improvements	\$41,364	\$39,297
Building and improvements	354,957	335,991
Manufacturing and laboratory equipment	136,432	124,564
Computer hardware and software	106,453	97,032
Furniture and equipment	16,465	13,717
Land improvements	4,557	4,106
Land	29,358	29,358
Construction-in-progress	180,220	108,340
	869,806	752,405
Less: Accumulated depreciation	(265,293)	(228,889)
Total property, plant and equipment, net	\$604,513	\$523,516

Construction in-process primarily includes costs related to the Company's significant in-process projects at its campus in San Rafael, California and manufacturing plant in Shanbally, Ireland.

Depreciation expense for the three and nine months ended September 30, 2015 was \$12.7 million and \$36.4 million, respectively, of which \$3.7 million and \$11.0 million, respectively, was capitalized into inventory. Depreciation expense for the three and nine months ended September 30, 2014 was \$11.2 million and \$31.3 million, respectively, of which \$2.6 million and \$8.0 million, respectively, was capitalized into inventory.

Capitalized interest related to the Company's property, plant and equipment purchases for each of the three and nine months ended September 30, 2015 and 2014 was insignificant.

## (12) SUPPLEMENTAL BALANCE SHEET INFORMATION

Inventory consisted of the following:

September 30,	December 31,
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	2015	2014
Raw materials	\$41,761	\$22,488
Work-in-process	137,751	114,393
Finished goods	82,588	62,571
Total inventory	\$262,100	\$199,452

Other Current Assets consisted of the following:

	September 30, 2015	December 31, 2014
Prepaid expenses	\$73,171	\$35,390
Short-term forward currency exchange contract assets	16,396	10,513
Promissory notes receivable, net	—	46,946
Restricted investments	7,346	2,354
Convertible promissory note conversion option	—	2,386
Other receivables	12,606	9,733
Other	5,811	4,513
Total other current assets	\$115,330	\$111,835

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Other Assets consisted of the following:

	September 30, 2015	December 31, 2014
Deposits	\$ 19,957	\$ 12,021
Deferred debt offering costs	9,259	11,763
Strategic investments	21,420	30,811
Long-term forward foreign currency exchange contract assets	3,642	5,387
Other	6,512	6,338
Total other assets	\$ 60,790	\$ 66,320

Accounts payable and accrued liabilities consisted of the following:

	September 30, 2015	December 31, 2014
Accounts payable and accrued operating expenses	\$ 162,926	\$ 139,513
Accrued compensation expense	62,674	45,479
Accrued vacation expense	15,450	12,540
Accrued rebates payable	27,357	14,859
Accrued royalties payable	9,886	9,050
Value added taxes payable	6,040	5,479
Other	11,867	4,924
Total accounts payable and accrued liabilities	\$ 296,200	\$ 231,844

## (13) CONVERTIBLE DEBT

The following table summarizes information regarding the Company's convertible debt:

	September 30, 2015	December 31, 2014
Convertible Notes due 2020, net of unamortized discount of	\$ 306,561	\$ 297,955

\$68,432 and \$77,045, at September 30, 2015 and		
December 31, 2014, respectively		
Convertible Notes due 2018, net of unamortized discount of		
\$45,376 and \$55,537, at September 30, 2015 and		
December 31, 2014, respectively		
Convertible Notes due 2017	329,604	319,463
Total convertible debt, net of unamortized discount	31,628	40,558
	\$667,793	\$657,976
Fair value of fixed rate convertible debt		
Convertible Notes due in 2020 <sup>(1)</sup>	\$503,706	\$456,360
Convertible Notes due in 2018 <sup>(1)</sup>	488,858	442,448
Convertible Notes due in 2017 <sup>(1)</sup>	163,806	180,984
Total	\$1,156,370	\$1,079,792

(1) The fair value of the Company's fixed rate convertible debt is based on open market trades and is classified as Level 1 in the fair value hierarchy.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Interest expense on the Company's convertible debt was comprised of the following:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Coupon interest	\$2,283	\$2,278	\$7,667	\$7,140
Amortization of issuance costs	823	828	2,471	2,505
Accretion of debt discount	6,341	6,012	18,773	17,800
Total interest expense on convertible debt	\$9,447	\$9,118	\$28,911	\$27,445

During the nine months ended September 30, 2015, the Company entered into separate agreements with three existing holders of its senior subordinated convertible notes due in 2017 (the 2017 Notes), pursuant to which such holders converted \$8.1 million in aggregate principal amount of the 2017 Notes into 399,469 shares of the Company's common stock. In addition to issuing the requisite number of shares of the Company's common stock, the Company also made varying cash payments to the holders totaling \$0.2 million in the aggregate, of which \$0.2 million was recognized in total as Debt Conversion Expense on the Condensed Consolidated Statements of Comprehensive Income (Loss) for the nine months ended September 30, 2015.

During the nine months ended September 30, 2014, the Company entered into two separate agreements with an existing holder of its 2017 Notes pursuant to which such holders converted \$16.5 million in aggregate principal amount of the 2017 Notes into 809,351 shares of the Company's common stock. In addition to issuing the requisite number of shares of the Company's common stock, the Company also made varying cash payments to the holder totaling \$0.7 million in the aggregate, of which \$0.7 million was recognized in total as Debt Conversion Expense on the Company's Condensed Consolidated Statement of Comprehensive Income (Loss) for the nine months ended September 30, 2014.

See Note 13 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014 for additional information related to the Company's Convertible Debt.

## (14) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

The Company uses forward foreign currency exchange contracts to hedge certain operational exposures resulting from potential changes in foreign currency exchange rates. Such exposures result from portions of the Company's forecasted revenues and operating expenses being denominated in currencies other than the U.S. dollar, primarily the Euro, the British Pound and the Brazilian Real.

The Company designates certain of these forward foreign currency exchange contracts as hedging instruments and enters into some forward foreign currency exchange contracts that are considered to be economic hedges that are not designated as hedging instruments. Whether designated or undesignated, these forward foreign currency exchange contracts protect against the reduction in value of forecasted foreign currency cash flows resulting from product revenues, royalty revenues, operating expenses and asset or liability positions designated in currencies other than the U.S. dollar. The fair values of forward foreign currency exchange contracts are estimated using current exchange rates and interest rates, and take into consideration the current creditworthiness of the counterparties or the Company, as applicable. Information regarding the specific instruments used by the Company to hedge its exposure to foreign currency exchange rate fluctuations is provided below. See Note 15 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of forward foreign currency exchange contracts.

At September 30, 2015, the Company had 215 forward foreign currency exchange contracts outstanding to sell a total of 296.4 million Euros, 160 forward foreign currency exchange contracts outstanding to purchase 155.1 million Euros and one forward foreign currency exchange contract to sell 70.0 million Reais with expiration dates ranging from October 2015 through September 2018. These hedges were entered into in order to protect against the fluctuations in revenue and operating expenses associated with Euro-denominated cash flows. The Company has formally designated these forward foreign currency exchange contracts as cash flow hedges and expects them to be highly effective in offsetting fluctuations in revenues and operating expenses denominated in Euros related to changes in foreign currency exchange rates.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The Company also enters into forward foreign currency exchange contracts that are not designated as hedges for accounting purposes. The changes in fair value of these forward foreign currency exchange contracts are included as a part of SG&A expense in the Company's Condensed Consolidated Statements of Comprehensive Income (Loss). At September 30, 2015, the Company had one outstanding forward foreign currency exchange contract to sell 58.0 million Euros, one outstanding forward foreign currency contract to purchase 7.0 million Euros and one outstanding forward foreign currency exchange contract to sell 7.1 million British Pounds, which were not designated as hedges for accounting purposes and matured on October 30, 2015.

The maximum length of time over which the Company is hedging its exposure to the reduction in value of forecasted foreign currency revenues through forward foreign currency exchange contracts is through September 2018. Over the next twelve months, the Company expects to reclassify \$15.1 million from accumulated other comprehensive income to earnings as the forecasted revenue transactions occur.

The fair value carrying amounts of the Company's derivative instruments were as follows:

	Asset Derivatives September 30, 2015		Liability Derivatives September 30, 2015	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 16,326	Accounts payable and accrued liabilities	\$ 2,502
Forward foreign currency exchange contracts	Other assets	3,642	Other long- term liabilities	3,186
Total		19,968		5,688
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	70	Accounts payable and accrued liabilities	131
	Other assets	—	Other long- term liabilities	—
Total		70		131
Total value of derivative contracts		\$ 20,038		\$ 5,819

	Asset Derivatives December 31, 2014		Liability Derivatives December 31, 2014	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				

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Forward foreign currency exchange contracts	Other current assets	\$ 10,206	Accounts payable and accrued liabilities	\$ —
Forward foreign currency exchange contracts	Other assets	5,387	Other long- term liabilities	—
Total		15,593		—
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	307	Accounts payable and accrued liabilities	12
Total		307		12
Total value of derivative contracts		\$ 15,900		\$ 12



## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The effect of the Company's derivative instruments on the Condensed Consolidated Financial Statements for the three and nine months ended September 30, 2015 and 2014 was as follows:

	Forward Foreign Currency Exchange Contracts			
	Three Months Ended		Nine Months Ended	
	September 30, 2015	September 30, 2014	September 30, 2015	September 30, 2014
<b>Derivatives Designated as Hedging Instruments:</b>				
Net gain recognized in Other Comprehensive Income (OCI) <sup>(1)</sup>	\$ 3,126	\$ 6,247	\$ 14,191	\$ 9,395
Net gain (loss) reclassified from accumulated OCI into earnings <sup>(2)</sup>	5,187	359	15,084	(662 )
Net loss recognized in net income (loss) <sup>(3)</sup>	(264 )	(179 )	(404 )	(499 )
<b>Derivatives Not Designated as Hedging Instruments:</b>				
Net gain (loss) recognized in net income (loss) <sup>(4)</sup>	\$ (514 )	\$ 4,365	\$ 6,052	\$ 4,861

(1) Net change in the fair value of the effective portion classified as OCI.

(2) Effective portion classified as net product revenue or SG&A expense.

(3) Ineffective portion and amount excluded from effectiveness testing classified as SG&A expense.

(4) Classified as SG&A expense.

At September 30, 2015 and December 31, 2014, accumulated other comprehensive income before taxes associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a gain of \$15.0 million and a gain of \$15.9 million, respectively.

The Company is exposed to counterparty credit risk on all of its derivative financial instruments. The Company has established and maintains strict counterparty credit guidelines and enters into hedges only with financial institutions that are investment grade or better to minimize the Company's exposure to potential defaults. The Company does not require collateral to be pledged under these agreements.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

## (15) FAIR VALUE MEASUREMENTS

The Company measures certain financial assets and liabilities at fair value on a recurring basis, including available-for-sale fixed income securities and foreign currency derivatives. The tables below present the fair value of these financial assets and liabilities determined using the following input levels.

Fair Value Measurements at September 30, 2015				
Quoted Price in				
	Active Markets	Significant Other	Significant	
	For Identical	Observable	Unobservable	
	Assets	Inputs	Inputs	
	(Level 1)	(Level 2)	(Level 3)	Total
<b>Assets:</b>				
<b>Cash and cash equivalents:</b>				
Overnight deposits	\$277,738	\$ —	\$ —	\$277,738
Money market instruments	—	98,608	—	98,608
<b>Total cash and cash equivalents</b>	<b>277,738</b>	<b>98,608</b>	<b>—</b>	<b>376,346</b>
<b>Available-for-sale securities:</b>				
<b>Short-term:</b>				
Certificates of deposit	—	50,138	—	50,138
Corporate debt securities	—	57,446	—	57,446
Commercial paper	—	60,892	—	60,892
U.S. government agency securities	—	73,508	—	73,508
<b>Long-term:</b>				
Certificates of deposit	—	7,550	—	7,550
Corporate debt securities	—	355,968	—	355,968
U.S. government agency securities	—	150,733	—	150,733
Greek government-issued bonds	—	130	—	130
<b>Total available-for-sale securities</b>	<b>—</b>	<b>756,365</b>	<b>—</b>	<b>756,365</b>
<b>Other Current Assets:</b>				
Nonqualified Deferred Compensation Plan assets	—	348	—	348
Forward foreign currency exchange contract <sup>(1)</sup>	—	16,396	—	16,396
Restricted investments <sup>(2)</sup>	—	7,346	—	7,346
<b>Total other current assets</b>	<b>—</b>	<b>24,090</b>	<b>—</b>	<b>24,090</b>
<b>Other Assets:</b>				

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Nonqualified Deferred Compensation Plan assets	—	5,989	—	5,989
Forward foreign currency exchange contract <sup>(1)</sup>	—	3,642	—	3,642
Strategic investment <sup>(4)</sup>	21,420	—	—	21,420
Total other assets	21,420	9,631	—	31,051
Total assets	\$299,158	\$ 888,694	\$ —	\$1,187,852
<b>Liabilities:</b>				
<b>Current Liabilities:</b>				
Nonqualified Deferred Compensation Plan liability	\$1,783	\$ 348	\$ —	\$2,131
Forward foreign currency exchange contract <sup>(1)</sup>	—	2,633	—	2,633
Contingent acquisition consideration payable	—	—	94,291	94,291
Total current liabilities	1,783	2,981	94,291	99,055
<b>Other long-term liabilities:</b>				
Nonqualified Deferred Compensation Plan liability	24,341	5,989	—	30,330
Forward foreign currency exchange contract <sup>(1)</sup>	—	3,186	—	3,186
Contingent acquisition consideration payable	—	—	34,874	34,874
Total other long-term liabilities	24,341	9,175	34,874	68,390
Total liabilities	\$26,124	\$ 12,156	\$ 129,165	\$167,445

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

	Fair Value Measurements at December 31, 2014			Total
	Quoted Price in			
	Active	Significant Other	Significant	
	For	Observable	Unobservable	
Identical	Inputs	Inputs		
Assets	Inputs	Inputs		
(Level 1)	(Level 2)	(Level 3)		
<b>Assets:</b>				
Cash and cash equivalents:				
Overnight deposits	\$225,159	\$ —	\$ —	\$225,159
Money market instruments	—	650,327	—	650,327
Total cash and cash equivalents	225,159	650,327	—	875,486
Available-for-sale securities:				
Short-term:				
Certificates of deposit	—	54,174	—	54,174
Corporate debt securities	—	15,532	—	15,532
Long-term:				
Certificates of deposit	—	18,129	—	18,129
Corporate debt securities	—	79,604	—	79,604
Greek government-issued bonds	—	123	—	123
Total available-for-sale securities	—	167,562	—	167,562
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	—	514	—	514
Forward foreign currency exchange contract <sup>(1)</sup>	—	10,513	—	10,513
Restricted investments <sup>(2)</sup>	—	2,354	—	2,354
Embedded derivative <sup>(3)</sup>	—	—	2,386	2,386
Total other current assets	—	13,381	2,386	15,767
Other Assets:				
Nonqualified Deferred Compensation Plan assets	—	5,112	—	5,112
Restricted investments <sup>(2)</sup>	—	5,387	—	5,387
Strategic investment <sup>(4)</sup>	30,811	—	—	30,811
Total other assets	30,811	10,499	—	41,310
Total assets	\$255,970	\$ 841,769	\$ 2,386	\$1,100,125
<b>Liabilities:</b>				
Current Liabilities:				
Nonqualified Deferred Compensation Plan liability	\$1,790	\$ 514	\$ —	\$2,304
Forward foreign currency exchange contract <sup>(1)</sup>	—	12	—	12
Contingent acquisition consideration payable	—	—	3,895	3,895

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Total current liabilities	1,790	526	3,895	6,211
Other long-term liabilities:				
Nonqualified Deferred Compensation Plan liability	18,453	5,112	—	23,565
Contingent acquisition consideration payable	—	—	38,767	38,767
Total other long-term liabilities	18,453	5,112	38,767	62,332
Total liabilities	\$20,243	\$ 5,638	\$ 42,662	\$68,543

- (1) See Note 14 to these Condensed Consolidated Financial Statements for further information regarding the derivative instruments.
- (2) The restricted investments at September 30, 2015 and December 31, 2014 secure the Company's irrevocable standby letter of credit obtained in connection with certain commercial agreements.
- (3) The embedded derivative at December 31, 2014 represents the fair value of the conversion feature of a promissory note that may be settled in the issuer's underlying shares.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(4)The Company has investments in marketable equity securities measured using quoted prices in an active market that are considered strategic investments. See Note 8 to these Condensed Consolidated Financial Statements for additional discussion regarding the Company's strategic investments.

There were no transfers between levels during the three and nine months ended September 30, 2015.

The Company's Level 2 securities are valued using third-party pricing sources. The pricing services utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs.

The Company validates the prices provided by its third-party pricing services by understanding the models used, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming those securities traded in active markets. See Note 8 to these Condensed Consolidated Financial Statements for further information regarding the Company's financial instruments.

Liabilities measured at fair value using Level 3 inputs were comprised of contingent acquisition consideration payable and asset retirement obligations.

The Company's contingent acquisition consideration payable is estimated using a probability-based income approach utilizing an appropriate discount rate. Key assumptions used by management to estimate the fair value of contingent acquisition consideration payable include estimated probabilities, the estimated timing of when a milestone may be attained and assumed discount periods and rates. Subsequent changes in the fair value of the contingent acquisition consideration payable, resulting from management's revision of key assumptions, will be recorded in Intangible Asset Amortization and Contingent Consideration in the Company's Condensed Consolidated Statements of Comprehensive Income (Loss). The probability-based income approach used by management to estimate the fair value of the contingent acquisition consideration is most sensitive to changes in the estimated probabilities.

Contingent acquisition consideration payable at December 31, 2014	\$42,662
Addition of contingent consideration payable related to	
the Prosensa acquisition (CVR)	71,402
Changes in the fair value of contingent acquisition	
consideration payable	15,101
Contingent acquisition consideration payable at September 30, 2015	\$129,165

Under certain of the Company's lease agreements, the Company is contractually obligated to return leased space to its original condition upon termination of the lease agreement. The Company records an asset retirement obligation liability and a corresponding capital asset in an amount equal to the estimated fair value of the obligation when

estimable. In subsequent periods, for each such lease, the Company records Interest Expense to accrete the asset retirement obligation liability to full value and depreciates each capitalized asset retirement obligation asset, both over the term of the associated lease agreement.

Asset retirement obligations at December 31, 2014	\$3,765
Accretion expense	109
Additions	748
Payments	(29 )
Asset retirement obligations at September 30, 2015	\$4,593

The Company acquired intangible assets as a result of various business acquisitions. The estimated fair value of these long-lived assets was measured using Level 3 inputs as of the acquisition date.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

## (16) STOCK-BASED COMPENSATION

The Company's stock-based compensation plans include the Amended and Restated 2006 Share Incentive Plan (the Share Incentive Plan), the ESPP, the 2014 Inducement Plan and the 2012 Inducement Plan. The 2012 Inducement Plan expired in May 2013 and the 2014 Inducement Plan expired in June 2015. The Company's stock-based compensation plans are administered by the Compensation Committee of the Company's Board of Directors (the Board), which selects persons to receive awards and determines the number of shares subject to each award and the terms, conditions, performance measures and other provisions of the awards. See Note 16 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014 for additional information related to these stock-based compensation plans.

## Determining the Fair Value of Stock Options and Stock Purchase Rights

The fair value of each option award is estimated on the date of grant using the Black-Scholes valuation model and the assumptions noted in the tables below. The expected life of options is based on observed historical exercise patterns. Groups of employees that have similar historical exercise patterns were considered separately for valuation purposes, and as of September 30, 2015 the Company has identified two groups with distinctly different exercise patterns. The two groups identified are executive and non-executive employees. The executive employee group has a history of holding options for longer periods than non-executive employees. The expected volatility of stock options is based upon the weighted average of the historical volatility of the Company's common stock and the implied volatility of traded options on the Company's common stock for fiscal periods in which there is sufficient trading volume in options on the Company's common stock. The risk-free interest rate is based on the implied yield on a U.S. Treasury zero-coupon issue with a remaining term equal to the expected term of the option. The dividend yield reflects that the Company has not paid any cash dividends since inception and does not intend to pay any cash dividends in the foreseeable future. The assumptions used to estimate the per share fair value of stock options granted under the 2012 Inducement Plan, the 2014 Inducement Plan and the Share Incentive Plan were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Expected volatility	41 – 44%	44 – 45%	39 – 45%	44 – 45%
Dividend yield	0.0%	0.0%	0.0%	0.0%
Expected life	6.9 – 8.0 years	6.9 years	6.4 – 8.0 years	6.9 years
Risk-free interest rate	1.9– 2.1%	2.0 – 2.2%	1.5– 2.2%	2.0 – 2.3%

During the nine months ended September 30, 2015, the Company granted 718,670 options with a weighted average fair value of \$56.86 per option.

The Company did not issue any new stock purchase rights under the ESPP during the three months ended September 30, 2015.



Restricted Stock Unit Awards with Service-Based Vesting Conditions

Restricted stock units (RSUs) are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The Company expenses the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse. During the nine months ended September 30, 2015, the Company granted 1,098,715 RSUs with a weighted average fair market value of \$120.55 per share.

Restricted Stock Unit Awards with Performance and Market-Based Vesting Conditions

During 2012 and 2011, pursuant to the approval of the Board, the Company granted 860,000 RSU awards with performance and market-based vesting conditions (the 2011/2012 Base RSUs) under the Share Incentive Plan and the 2012 Inducement Plan to certain executive officers. As of September 30, 2015, the 2011/2012 Base RSUs had a weighted-average grant date fair value of \$34.66. The 2011/2012 Base RSUs will vest upon the achievement of specific performance goals (the Earned RSUs). The number of RSUs that will be awarded from the Earned RSUs will be calculated by multiplying the Earned RSUs by the Total Shareholder Return multiplier which could range from 75% to 125%.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Stock-based compensation expense for this award will be recognized over the remaining service period beginning in the period the Company determines the strategic performance goal or goals is probable of achievement. During the fourth quarter of 2014, management concluded that the achievement of the 2015 revenue performance goal was probable and began recognizing compensation expense related to the RSUs allocated to the revenue performance goal. During 2013, management concluded that regulatory approval of Vimizim was probable and began recognizing compensation expense related to the performance 2011/2012 Base RSUs allocated to the Vimizim performance goal. The Company recognized compensation expense of \$1.5 million and \$4.6 million for these awards during the three and nine months ended September 30, 2015, respectively. For the three and nine months ended September 30, 2014, the Company recognized compensation expense related to these awards of \$0.7 million and \$1.6 million, respectively.

## Restricted Stock Unit Awards with Performance Conditions

On March 3, 2015, pursuant to Board approval, the Company granted 58,300 RSU awards with performance-vesting conditions (the 2015 Base RSUs) under the Share Incentive Plan to certain executive officers. The vesting of the 2015 Base RSUs under this specific grant is contingent upon the achievement of a 2015 revenue target and a three-year service period. The number of RSUs that will be awarded from the 2015 Base RSUs upon achievement of the performance condition will be calculated by multiplying the 2015 Base RSUs by a revenue multiplier (determined based on the Company's performance against the revenue target) which could range between 80% to 120%. The maximum number of RSUs that could vest if the performance condition is achieved and a revenue multiplier of 120% is applied is 69,960.

Stock-based compensation for these awards will be recognized over the service period beginning in the period the Company determines it is probable that the revenue target will be achieved. The cost of the 2015 Base RSUs was determined to be \$108.36 per RSU, based on the fair value of the common stock underlying the 2015 Base RSUs on the grant date. Accordingly, because the Company's management determined that attainment of the revenue target is probable, the Company recognized \$0.5 million and \$1.3 million of compensation expense related to these awards during the three and nine months ended September 30, 2015, respectively.

Compensation expense included in the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) for all stock-based compensation arrangements was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Cost of sales	\$1,386	\$1,180	\$4,484	\$3,807
R&D	12,578	8,279	34,972	22,300
SG&A	14,794	10,545	41,503	27,452
Total stock-based compensation expense	\$28,758	\$20,004	\$80,959	\$53,559

Stock-based compensation of \$8.3 million and \$5.7 million was capitalized into inventory, for the nine months ended September 30, 2015 and 2014, respectively. Capitalized stock-based compensation is recognized as cost of sales when the related product is sold.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

## (17) COMPREHENSIVE INCOME (LOSS)

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income/(Loss) (AOCI) and their effect on the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2015 and 2014.

Details about AOCI Components	Amount Reclassified from AOCI Gain (Loss)				Consolidated Statement of Comprehensive Income (Loss) Classification
	Three Months Ended September 30,		Nine Months Ended September 30,		
	2015	2014	2015	2014	
Gains (loss) on cash flow hedges:					
Forward foreign currency exchange					
contracts	\$4,411	\$562	\$13,660	\$(1,035)	Net product revenues
Forward foreign currency exchange					
contracts	776	—	1,424	—	SG&A
Gain on sale of available-for-sale					
investment	14	—	3,036	—	Other income (expense)
Less income tax effect of the above	5	203	1,098	(373)	Provision for (benefit from) income taxes
	\$5,196	\$359	\$17,022	\$(662)	Net income (loss)

The following tables summarize changes in the accumulated balances for each component of AOCI, including current period other comprehensive income and reclassifications out of AOCI, for the three and nine months ended September 30, 2015 and 2014.

	Three Months Ended September 30, 2015		
	Before Tax	Tax (Expense)	Net-of-Tax Amount
AOCI balance at June 30, 2015	\$41,156	\$(8,695)	\$32,461
Foreign currency translation adjustment	—	—	—

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Unrealized gain on available-for-sale securities:			
Unrealized holding loss	(8,158 )	2,951	(5,207 )
Less: reclassification adjustment for gain realized in net loss	14	(5 )	9
Net unrealized holding loss	(8,172 )	2,956	(5,216 )
Net unrealized holding gain on cash flow hedges:			
Unrealized holding loss	3,126	—	3,126
Less: reclassification adjustment for gain realized in net loss	5,187	—	5,187
Net unrealized holding loss	(2,061 )	—	(2,061 )
Other comprehensive income	(10,233)	2,956	(7,277 )
AOCI balance at September 30, 2015	\$30,923	\$ (5,739 )	\$ 25,184

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

	Nine Months Ended September 30, 2015		
	Before Tax	Tax (Expense)	Net-of-Tax
	Amount	Benefit	Amount
AOCI balance at December 31, 2014	\$33,984	\$ (6,518 )	\$ 27,466
Foreign currency translation adjustment	3	—	3
Unrealized gain on available-for-sale securities:			
Unrealized holding gain	865	(319 )	546
Less: reclassification adjustment for gain realized in net loss	3,036	(1,098 )	1,938
Net unrealized holding gain	(2,171 )	779	(1,392 )
Net unrealized holding gain on cash flow hedges:			
Unrealized holding gain	14,191	—	14,191
Less: reclassification adjustment for gain realized in net loss	15,084	—	15,084
Net unrealized holding gain	(893 )	—	(893 )
Other comprehensive income	(3,061 )	779	(2,282 )
AOCI balance at September 30, 2015	\$30,923	\$ (5,739 )	\$ 25,184
	Three Months Ended September 30, 2014		
	Before Tax	Tax (Expense)	Net-of-Tax
	Amount	Benefit	Amount
AOCI balance at June 30, 2014	\$15,491	\$ (5,550 )	\$ 9,941
Foreign currency translation adjustment	(3 )	—	(3 )
Unrealized gain on available-for-sale securities:			
Unrealized holding gain	4,138	(1,493 )	2,645
Less: reclassification adjustment for gain realized in net income	—	—	—
Net unrealized holding gain	4,138	(1,493 )	2,645
Net unrealized holding gain on cash flow hedges:			
Unrealized holding gain	9,768	(3,521 )	6,247
Less: reclassification adjustment for loss realized in net income	562	(203 )	359
Net unrealized holding gain	10,330	(3,724 )	6,606
Other comprehensive income	14,465	(5,217 )	9,248
AOCI balance at September 30, 2014	\$29,956	\$ (10,767 )	\$ 19,189
	Nine Months Ended September 30, 2014		
			Net-of-Tax

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	Before Tax	Tax (Expense)	Amount
	Amount	Benefit	
AOCI balance at December 31, 2013	\$7,756	\$ (2,738 )	\$ 5,018
Foreign currency translation adjustment	(36 )	—	(36 )
Unrealized gain on available-for-sale securities:			
Unrealized holding gain	8,580	(3,106 )	5,474
Less: reclassification adjustment for gain realized in net loss	—	—	—
Net unrealized holding gain	8,580	(3,106 )	5,474
Net unrealized holding gain on cash flow hedges:			
Unrealized holding gain	14,691	(5,296 )	9,395
Less: reclassification adjustment for loss realized in net loss	(1,035 )	373	(662 )
Net unrealized holding gain	13,656	(4,923 )	8,733
Other comprehensive income	22,200	(8,029 )	14,171
AOCI balance at September 30, 2014	\$29,956	\$ (10,767 )	\$ 19,189

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

## (18) REVENUE AND CREDIT CONCENTRATIONS

Net Product Revenue—The Company considers there to be revenue concentration risks for regions where net product revenue exceeds ten percent (10%) of consolidated net product revenue. The concentration of the Company's net product revenue within the regions below may have a material adverse effect on the Company's revenue and results of operations if sales in the respective regions experience difficulties.

The table below summarizes consolidated net product revenue concentrations based on patient location for Vimizim, Naglazyme, Kuvan and Firdapse which are sold directly by the Company and global sales of Aldurazyme which is marketed by Genzyme Corporation (Genzyme). Genzyme is the Company's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. Net product revenues from Genzyme are comprised of royalties on worldwide net Aldurazyme sales and incremental product transfer revenues.

	Three Months Ended September 30, 2015		2014		Nine Months Ended September 30, 2015		2014	
Net product revenue marketed by the Company								
United States	44	%	42	%	38	%	37	%
Europe	21	%	22	%	20	%	19	%
Latin America	12	%	8	%	19	%	15	%
Rest of world	13	%	15	%	14	%	16	%
Total net product revenue marketed by the Company	90	%	87	%	91	%	87	%
Aldurazyme net product revenues marketed by Genzyme	10	%	13	%	9	%	13	%
Total net product revenue	100	%	100	%	100	%	100	%

The following table illustrates the percentage of the Company's consolidated net product revenue attributed to the Company's five largest customers.

	Three Months Ended September 30, 2015		2014		Nine Months Ended September 30, 2015		2014	
Customer A	15	%	17	%	14	%	15	%



Customer B <sup>(1)</sup>	9 %	13 %	8 %	13 %
Customer C	6 %	4 %	13 %	11 %
Customer D	9 %	13 %	11 %	11 %
Customer E	13 %	—	5 %	—
Total	52 %	47 %	51 %	50 %

(1) Genzyme is the Company's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. Net product revenues from Genzyme are comprised of royalties on worldwide net Aldurazyme sales and incremental product transfer revenue.

On a consolidated basis, the Company's two largest customers accounted for 31% and 17% of the September 30, 2015 accounts receivable balance, respectively, compared to December 31, 2014 when the two largest customers accounted for 42% and 18% of the accounts receivable balance, respectively. As of September 30, 2015 and December 31, 2014, accounts receivable for the Company's largest customer balance included \$23.9 million and \$34.5 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme. The Company does not require collateral from its customers, but does perform periodic credit evaluations of its customers' financial condition and requires immediate payment in certain circumstances.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The Company is subject to credit risk from accounts receivable related to product sales. The majority of the Company's trade accounts receivable arises from product sales in the U.S. and the EU. The Company's product sales to government-owned or government-funded customers in certain European countries, including Italy, Spain, Portugal, Greece and Russia, are subject to payment terms that are statutorily determined. Because these customers are government-owned or government-funded, the Company may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings or a default in these countries may decrease the likelihood that the Company will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to the Company's operating results. For each of the three and nine months ended September 30, 2015, the Company's net product revenues for these countries was 4%. Additionally, approximately 8% of the Company's outstanding accounts receivable at September 30, 2015 related to such countries.

As of September 30, 2015, the Company's accounts receivable in certain European countries, specifically Greece, Italy, Portugal, Spain and Russia, totaled approximately \$12.5 million, of which \$8.1 million is current and \$2.1 million is less than 30 days past due.

The Company also sells its products in other countries that face economic crises and local currency devaluation. Although the Company has historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause customers in those countries to be unable to pay for the Company's products. The Company has not historically experienced a significant level of uncollected receivables and has received continued payments from its more aged accounts. The Company believes that the allowances for doubtful accounts related to these countries is adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries.

## (19) SEGMENT INFORMATION

The Company operates in one business segment, which primarily focuses on the development and commercialization of innovative biopharmaceuticals for serious diseases and medical conditions. All products are included in one segment because the majority of the Company's products have similar economic and other characteristics, including the nature of the products and production processes, type of customers, distribution methods and regulatory environment.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Net product revenue by product:				
Vimizim	\$65,106	\$25,239	\$169,608	\$40,389

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Naglazyme	54,131	67,511	243,386	245,954
Kuvan	64,219	53,438	174,501	145,578
Aldurazyme	20,509	22,553	58,991	64,727
Firdapse	3,802	4,675	11,616	14,016
Total net product revenue	\$207,767	\$173,416	\$658,102	\$510,664

Net product revenue is based on patient location for Vimizim, Naglazyme, Kuvan and Firdapse and Genzyme's headquarters for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties earned by the Company on Genzyme's net sales are included in the U.S. region, as the transactions are with Genzyme, whose headquarters are located in the U.S.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The following table summarizes total revenues from external customers and collaborative partners by geographic region.

	Three Months Ended		Nine Months Ended	
	September 30, 2015	2014	September 30, 2015	2014
Total revenues by geographic region:				
United States	\$ 112,254	\$ 98,070	\$ 310,209	\$ 259,738
Europe	42,822	39,743	127,796	102,808
Latin America	25,588	14,292	127,147	78,116
Rest of world	28,240	24,444	96,807	78,554
Total revenues	\$ 208,904	\$ 176,549	\$ 661,959	\$ 519,216

## (20) COMMITMENTS AND CONTINGENCIES

## Contingencies

From time to time the Company is involved in legal actions arising in the normal course of its business. The most significant of these actions are described below.

The process of resolving matters through litigation or other means is inherently uncertain and it is possible that an unfavorable resolution of these matters could adversely affect the Company, its results of operations, financial condition and cash flows. The Company's general practice is to expense legal fees as services are rendered in connection with legal matters, and to accrue for liabilities when losses are probable and reasonably estimable.

## Paragraph IV Notices

As previously disclosed, the Company received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying the Company that DRL has filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of the Company's patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Additionally, the Company received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying the Company that Par has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of the Company's patents listed in the Orange Book. Together with Merck & Cie, the Company filed lawsuits against both DRL and Par in the United States District Court for the District of New Jersey alleging patent infringement for the Company's patents relating to Kuvan, triggering the automatic 30-month stay on the approval of each ANDA. In response, DRL and Par alleged, inter alia, that the asserted patents are not

infringed and/or are invalid.

In September 2015, the Company entered into a settlement agreement with DRL that resolved the patent litigation with DRL in the United States related to Kuvan (sapropterin dihydrochloride) 100 mg oral tablets. Under the terms of the settlement, the Company will grant DRL a non-exclusive license to its Kuvan related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances. The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending. The Court has not yet set a date for a claim construction hearing or trial in the litigation against Par.

#### Contingent Payments

As of September 30, 2015, the Company is subject to contingent payments totaling approximately \$763.0 million upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, \$160.0 million relates to Prosensa and \$22.9 million relates to programs that are no longer being developed.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

As of September 30, 2015, \$129.2 million of the \$763.0 million of contingent payments noted above is recorded on the Company's Condensed Balances Sheets in contingent acquisition consideration payable, of which \$94.3 million is expected to be paid in the next twelve months.

In the normal course of business, the Company enters into various firm purchase commitments primarily related to research and development and certain inventory related items. As of September 30, 2015, these commitments for the next five years were approximately \$58.9 million. These amounts primarily relate to active pharmaceutical ingredients and represent minimum purchase requirements and post-marketing commitments related to the Company's approved products.

(21) SUBSEQUENT EVENTS

On October 1, 2015, the Company announced that it had entered into a definitive agreement to acquire all global rights to Kuvan and pegvaliase from Ares Trading, S.A. (Merck Serono), with the exception of Kuvan in Japan, in exchange for an upfront payment of €340.0 million and up to and additional €185.0 million if certain sales and regulatory milestones are attained. Previously, the Company had exclusive rights to Kuvan in the U.S. and Canada and pegvaliase in the U.S. and Japan. Under the terms of the agreement, the Company will have exclusive worldwide rights to Kuvan and pegvaliase with the exception of Kuvan in Japan. The closing of the Merck Serono transaction is subject to customary closing conditions, including regulatory approvals, and the Company expects to close the transaction in January 2016.

On October 6, 2015, the Company completed the sale of talazoparib to Medivation. Pursuant to the Asset Purchase Agreement, Medivation paid the Company an upfront payment of \$410.0 million upon the closing of the transaction. In addition, contingent upon the successful development and commercialization of talazoparib, Medivation will pay the Company milestone payments of up to \$160.0 million and mid-single digit percentage royalties on net sales of talazoparib.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations  
Forward-Looking Statements

This Quarterly Report on Form 10-Q contains "forward-looking statements" as defined under securities laws. Many of these statements can be identified by the use of terminology such as "believes," "expects," "anticipates," "plans," "may," "will," "projects," "continues," "estimates," "potential," "opportunity" or the negative versions of these terms and other similar expressions. These forward-looking statements may be found in "Overview," of this Item 2 and other sections of this Quarterly Report on Form 10-Q. Our actual results or experience could differ significantly from the forward-looking statements. Factors that could cause or contribute to these differences include those discussed in "Risk Factors," in Part II, Item 1A of this Quarterly Report on Form 10-Q as well as information provided elsewhere in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2014, which was filed with the Securities and Exchange Commission (the SEC) on March 2, 2015. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these types of forward-looking statements, which speak only as of the date that they were made. These forward-looking statements are based on the beliefs and assumptions of our management based on information currently available to management and should be considered in connection with any written or oral forward-looking statements that we may issue in the future as well as other cautionary statements we have made and may make. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Quarterly Report on Form 10-Q to reflect later events or circumstances or the occurrence of unanticipated events.

The following discussion of our financial condition and results of operations should be read in conjunction with our Condensed Consolidated Financial Statements and the related Notes thereto included elsewhere in this Quarterly Report on Form 10-Q.

#### Overview

We develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. We select product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products.

Our product portfolio is comprised of five approved products and multiple clinical and pre-clinical product candidates. Our approved products are Vimizim (elosulfase alfa), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

#### Business Highlights

During the nine months ended September 30, 2015, we continued to grow our commercial business and advance our product pipeline. We believe that the combination of our internal research programs, acquisitions and partnerships will allow us to continue develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. Below is a summary of our recent key accomplishments:

- We entered into a definitive agreement to acquire all global rights to Kuvan and pegvaliase from Ares Trading, S.A. (Merck Serono), with the exception of Kuvan in Japan. Under the terms of the agreement, we will provide Merck Serono with an upfront payment of €340.0 million. We have also agreed to pay Merck Serono additional consideration in future periods up to €185.0 million (undiscounted) in milestone payments if certain pegvaliase regulatory and sales milestones are attained. Previously, we had exclusive rights to Kuvan in the United States (U.S.) and Canada and to pegvaliase in the U.S. and Japan. Under the terms of the agreement, we will have exclusive worldwide rights to Kuvan and pegvaliase with the exception of Kuvan in Japan. The closing of the Merck Serono transaction is subject to the customary closing conditions, including regulatory approval, and we expect to close the

transaction in January 2016.

- We entered into an asset purchase agreement with Medivation, Inc. (Medivation), under which Medivation acquired the worldwide rights to talazoparib in October 2015 in exchange for an upfront payment of \$410.0 million and up to an additional \$160.0 million upon the achievement of regulatory and sales-based milestones and mid-single digit percentage royalties for talazoparib.
- We enrolled the first patient in our Phase 1/2 trial for BMN 270, an investigational gene therapy for the treatment of patients with Hemophilia A.

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

- We entered into a settlement agreement with Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL) that resolves the patent litigation with DRL in the U.S. related to our Kuvan 100mg oral tablets. Under the terms of the settlement, we will grant DRL a non-exclusive license to our Kuvan related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances.
- In January 2015, we acquired Prosensa Holding N.V. (Prosensa) for a total purchase price of \$751.5 million. See Note 5 to our accompanying Condensed Consolidated Financial Statements for additional discussion.
- We announced the submission and acceptance of a U.S. Food and Drug Administration (the FDA) New Drug Application (NDA) for Kyndrisa, formerly referred to as drisapersen, and the submission and acceptance of a Marketing Authorization Application (MAA) for Kyndrisa with the European Medicines Agency (EMA).
- We received Rare Pediatric Disease Designation from the FDA for Kyndrisa.
- We reported total revenues of \$208.9 million and \$662.0 million for the three and nine months ended September 30, 2015, respectively, as compared to \$176.5 million and \$519.2 million for the three and nine months ended September 30, 2014, respectively.
- We announced positive results of a Phase 2 proof-of-concept and dose finding study of vosoritide, formerly referred to as BMN 111, for the treatment of achondroplasia.
- We shared the interim data from nine patients in the cerliponase alfa trial who have been followed for at least 15 months. Preliminary data suggest that treatment with cerliponase alfa may result in stabilization of CLN2 disease compared to the natural history based on a standardized measure of motor and language function. We expect to release the top line results from this Phase 1/2 trial in the first quarter of 2016.

## Financial Highlights

Key components of our results of operations include the following (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Total net product revenues	\$207.8	\$173.4	\$658.1	\$510.7
Cost of sales	36.7	29.9	109.4	83.9
Research & Development (R&D) expense	158.7	125.7	458.7	319.5
Selling, general and administrative (SG&A) expense	94.0	74.6	288.4	202.4
Intangible asset amortization and				
contingent consideration expense	1.3	2.6	17.5	15.0
Net income (loss)	(90.9 )	7.4	(240.4)	(64.2 )
Stock-based compensation expense	28.8	20.0	81.0	53.6

See "Results of Operations" below for a discussion of the detailed components and analysis of the amounts above.

Net product revenues were as follows (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Vimizim	\$65.1	\$25.2	\$169.6	\$40.4
Naglazyme	54.1	67.5	243.4	246.0
Kuvan	64.2	53.4	174.5	145.6
Aldurazyme	20.6	22.6	59.0	64.7
Firdapse	3.8	4.7	11.6	14.0
Total net product revenues	\$207.8	\$173.4	\$658.1	\$510.7

Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Cost of sales includes raw materials, personnel and facility and other costs associated with manufacturing Vimizim, Naglazyme and Aldurazyme at our production facility in Novato, California. Cost of sales also includes third-party manufacturing costs for the production of the active ingredient in Kuvan and Firdapse and third-party production costs related to final formulation and packaging services for all products and cost of royalties payable to third-parties for all products.

R&D expense includes costs associated with the research and development of product candidates and post-marketing research commitments related to our approved products. These costs primarily include preclinical and clinical studies, personnel and raw materials costs associated with manufacturing product candidates, quality control and assurance, research and development facilities and regulatory costs.

SG&A expense primarily includes expenses associated with the commercialization of approved products and general and administrative costs to support our operations. These expenses include: product marketing and sales operations personnel; corporate facility operating expenses; information technology expenses and depreciation; and core corporate support functions, including human resources, finance and legal, and other external corporate costs such as insurance, legal fees and other professional services.

Our cash, cash equivalents, short-term investments and long-term investments totaled \$1,132.7 million as of September 30, 2015, compared to \$1,043.1 million as of December 31, 2014. We have historically financed our operations primarily through our cash flows from operating activities and the issuance of common stock and convertible debt. We will be highly dependent on our net product revenue to supplement our current liquidity and fund our operations for the foreseeable future. We may in the future elect to supplement this with further debt or equity offerings or commercial borrowing, even after giving effect to our January 2015 equity offering. Further, depending on market conditions, our financial position and performance and other factors, we may in the future choose to use a portion of our cash or cash equivalents to repurchase our convertible debt or other securities. See "Financial Position, Liquidity and Capital Resources" below for a further discussion of our liquidity and capital resources.

#### Critical Accounting Policies and Estimates

In preparing our Condensed Consolidated Financial Statements in accordance with accounting principles generally accepted in the U.S. and pursuant to the rules and regulations promulgated by the SEC, we make assumptions, judgments and estimates that can have a significant impact on our net income/loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. We base our assumptions, judgments and estimates on experience and various other factors that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions. On a regular basis, we evaluate our assumptions, judgments and estimates. We also discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors.

We believe that the assumptions, judgments and estimates involved in the accounting for business combinations, contingent acquisition consideration payable, income taxes, long-lived assets, revenue recognition and inventory have the greatest impact on our Condensed Consolidated Financial Statements, so we consider these to be our critical accounting policies. Historically, our assumptions, judgments and estimates relative to our critical accounting policies have not differed materially from actual results.

There have been no significant changes to our critical accounting policies and estimates during the nine months ended September 30, 2015, as compared to the critical accounting policies and estimates disclosed in "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in our Annual Report on Form

10-K for the year ended December 31, 2014.

#### Recent Accounting Pronouncements

See Note 4 to our accompanying Condensed Consolidated Financial Statements for a full description of recent accounting pronouncements and our expectation of their impact, if any, on our results of operations and financial condition.

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## Results of Operations

## Net Income (Loss)

Our net loss for the three months ended September 30, 2015 was \$90.9 million, compared to a net income of \$7.4 million for the three months ended September 30, 2014. Our net loss for the nine months ended September 30, 2015 was \$240.4 million, compared to a net loss of \$64.2 million for the nine months ended September 30, 2014. The increase in net loss was primarily a result of the following (in millions):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Change	2015	2014	Change
Total revenues	\$208.9	\$176.5	\$32.4	\$662.0	\$519.2	\$142.8
Cost of Sales	36.7	29.9	6.8	109.4	83.9	25.5
R&D	158.7	125.7	33.0	458.7	319.5	139.2
SG&A	94.0	74.6	19.4	288.4	202.4	86.0
Intangible asset amortization and contingent consideration	1.3	2.6	(1.3 )	17.5	15.0	2.5
Gain on sale of intangible asset	—	(67.5 )	67.5	—	(67.5 )	67.5
Other, net	(8.6 )	(8.0 )	(0.6 )	(35.7 )	(25.1 )	(10.6 )
Provision for (benefit from) income taxes	0.5	(4.2 )	4.7	(7.3 )	5.0	(12.3 )
Net income (loss)	\$(90.9 )	\$7.4	\$(98.3 )	\$(240.4 )	\$(64.2 )	\$(176.2 )

See below for additional information related to the primary net loss fluctuations presented above, including details of our operating expense fluctuations.

## Net Product Revenues, Cost of Sales and Gross Profit

A summary of our various commercial products, including key metrics as of September 30, 2015, is provided below:

Commercial Products	Indication	Orphan Drug Exclusivity Expiration U.S.	Orphan Drug Exclusivity Expiration EU
Vimizim	MPS IV A <sup>(1)</sup>	2021	2024
Naglazyme	MPS VI <sup>(2)</sup>	Expired	Expired
Kuvan	PKU <sup>(3)</sup>	Expired	NA <sup>(4)</sup>
Aldurazyme <sup>(5)</sup>	MPS I <sup>(6)</sup>	Expired	Expired
Firdapse	LEMS <sup>(7)</sup>	NA <sup>(8)</sup>	2019

(1) Mucopolysaccharidosis IV Type A, or MPS IVA

(2) Mucopolysaccharidosis VI, or MPS VI

(3) Phenylketonuria, or PKU

(4) Merck Serono S.A. markets Kuvan in the EU.

(5) The Aldurazyme total product revenue noted above is the total product revenue recognized by us in accordance with the terms of our agreement with Genzyme Corporation.

(6) Mucopolysaccharidosis I, or MPS I

(7) Lambert Eaton Myasthenic Syndrome, or LEMS

(8) Firdapse has not received marketing approval in the U.S. and we have licensed the North American rights to develop and market Firdapse to a third party.

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Net product revenues by product were as follows (in millions):

	Three Months Ended			Nine Months Ended		
	September 30,			September 30,		
	2015	2014	Change	2015	2014	Change
Vimizim	\$65.1	\$25.2	\$39.9	\$169.6	\$40.4	\$129.2
Naglazyme	54.1	67.5	(13.4 )	243.4	246.0	(2.6 )
Kuvan	64.2	53.4	10.8	174.5	145.6	28.9
Aldurazyme	20.6	22.6	(2.0 )	59.0	64.7	(5.7 )
Firdapse	3.8	4.7	(0.9 )	11.6	14.0	(2.4 )
Total net product revenues	\$207.8	\$173.4	\$34.4	\$658.1	\$510.7	\$147.4

Net product revenues attributed to our collaboration with Genzyme Corporation (Genzyme) were as follows (in millions):

	Three Months Ended			Nine Months Ended		
	September 30,			September 30,		
	2015	2014	Change	2015	2014	Change
Aldurazyme revenue reported by Genzyme	\$53.8	\$54.3	\$ (0.5 )	\$163.7	\$172.5	\$ (8.8 )
Royalties earned from Genzyme	\$23.3	\$22.9	\$ 0.4	\$69.1	\$69.1	\$ —
Incremental (previously recognized)						
Aldurazyme product transfer revenue		(2.7 )	(0.3 )	(2.4 )	(10.1 )	(4.4 )
Total Aldurazyme net product revenues	\$20.6	\$22.6	\$ (2.0 )	\$59.0	\$64.7	\$ (5.7 )

The FDA and the EMA granted marketing approval for Vimizim in February 2014 and April 2014, respectively, and Vimizim subsequently received marketing approval in other countries. We began marketing Vimizim immediately following approval in each of these markets. Net product revenues for Vimizim for the three and nine months ended September 30, 2015 totaled \$65.1 million and \$169.6 million, respectively, compared to \$25.2 million and \$40.4 million for the three and nine months ended September 30, 2014, respectively. Vimizim net product revenues earned from customers based outside the U.S. during the three and nine months ended September 30, 2015 totaled \$45.0 million and \$114.0 million, respectively, compared to \$11.3 million and \$16.5 million during the three and nine months ended September 30, 2014, respectively. The increase in Vimizim net product revenues for the three and nine months ended September 30, 2015 was attributed to new patients initiating therapy. The impact of foreign currency exchange rates, net of hedging activity, on Vimizim sales denominated in currencies other than the U.S. dollar was negative by \$5.5 million and \$11.7 million for the three and nine months ended September 30, 2015, respectively. Vimizim gross margins were 84% for each of the three and nine months ended September 30, 2015, respectively, compared to gross margins of 87% and 88% for the three and nine months ended September 30, 2014, respectively. In future periods, we do not expect Vimizim gross margins to fluctuate significantly from the mid-eighties.

Net product revenues for Naglazyme for the three and nine months ended September 30, 2015 totaled \$54.1 million and \$243.4 million, respectively, compared to \$67.5 million and \$246.0 million for the three and nine months ended September 30, 2014, respectively. Naglazyme net product revenues earned from customers based outside the U.S. totaled \$42.9 million and \$212.0 million for the three and nine months ended September 30, 2015, respectively, compared to \$58.1 million and \$216.8 million for the three and nine months ended September 30, 2014, respectively.

The increase in Naglazyme net product revenues attributed to new patients initiating therapy was offset by the negative impact of foreign currency exchange rates and the timing of significant purchases from certain government entities. The impact of foreign currency exchange rates, net of hedge activity, on Naglazyme sales denominated in currencies other than the U.S. dollar was negative by \$3.0 million and \$9.7 million for the three and nine months ended September 30, 2015 compared to a positive impact of \$0.2 million and \$1.3 million for the three and nine months ended September 30, 2014, respectively. Naglazyme gross margins were 83% and 85% for the three and nine months ended September 30, 2015, respectively, compared to 85% and 86% for the three and nine months ended September 30, 2014, respectively. Naglazyme gross margins are not expected to fluctuate significantly in the future.



## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Net product revenue for Kuvan for the three and nine months ended September 30, 2015 totaled \$64.2 million and \$174.5 million, respectively, compared to \$53.4 million and \$145.6 million for the three and nine months ended September 30, 2014, respectively. The increase in Kuvan net product revenues was attributed to new patients initiating therapy. Kuvan gross margins were 83% for each of the three and nine months ended September 30, 2015, compared to 84% for each of the three and nine months ended September 30, 2014. Cost of goods sold for each of the three and nine months ended September 30, 2015 and 2014 reflect royalties paid to third-parties of approximately 10%. Kuvan gross margins are not expected to fluctuate significantly in the future. The royalties earned from Merck Serono's net sales of Kuvan for each of the three and nine months ended September 30, 2015 were \$0.6 million and \$1.7 million, respectively compared to \$0.5 million and \$1.7 million for the three and nine months ended September 30, 2014, respectively.

We own several patents that cover Kuvan and have listed those patents in conjunction with that product in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act, permits the FDA to approve abbreviated new drug applications (ANDA), for the generic versions of branded drugs. The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of the applicant's proposed generic product prior to the expiration of our Orange Book-listed patents to notify us of the application. Upon receipt of such a notice (a paragraph IV notice), the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the application for the generic version of our drug. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA's review of the ANDA may be completed. The discovery, trial and appeals process in such a lawsuit is costly, time consuming, and may result in generic competition if the ANDA applicant prevails. We have also received New Patient Population exclusivity for Kuvan (sapropterin dihydrochloride) that expires in October 2017, including pediatric exclusivity. Thus, depending on the label of a generic product, generic versions of Kuvan may be prohibited until October 2017.

As previously disclosed, we received a paragraph IV notice letter, dated October 3, 2014, from DRL, notifying us that DRL had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the Orange Book. Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the Orange Book. Together with Merck & Cie, we filed lawsuits against both DRL and Par in the U.S. District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan triggering the automatic 30 month stay on the approval of each ANDA. In response, DRL and Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

In September 2015, we entered into a settlement agreement with DRL that resolved patent litigation with DRL in the United States related to Kuvan (sapropterin dihydrochloride) 100 mg oral tablets. The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending. The Court has not yet set a date for a claim construction hearing or trial in the litigation against Par.

The filing of DRL's and Par's purported ANDAs in respect to Kuvan could have an adverse impact on our stock price, and litigation to enforce our patents is likely to cost a substantial amount and require significant management attention. If the patents covering Kuvan and its use are not upheld in litigation, or if Par is found to not infringe our asserted patents, the resulting generic competition following the expiration of regulatory exclusivity would have a material adverse effect on our revenue and results of operations. Moreover, generic competition from DRL following

the settlement described above could have a material adverse effect on our revenue and results of operations.

Net product revenue for Aldurazyme for the three and nine months ended September 30, 2015 totaled \$20.6 million and \$59.0 million, respectively, compared to \$22.6 million and \$64.7 million for the three and nine months ended September 30, 2014, respectively. The decrease in Aldurazyme net product revenues was primarily attributed to a decrease in Genzyme reported Aldurazyme sales. Aldurazyme gross margins were 75% and 77% for the three and nine months ended September 30, 2015, respectively, compared to 69% and 71% for the three and nine months ended September 30, 2014, respectively. Aldurazyme gross margins reflect the profit earned on royalty revenue and net incremental product transfer revenue. Aldurazyme gross margins are expected to fluctuate depending on the mix of royalty revenue, from which we earn higher gross profit, and product transfer revenue, from which we earn lower gross profit.

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## Cost of Sales

Total cost of sales for the three and nine months ended September 30, 2015 were \$36.7 million and \$109.4 million, respectively, compared to \$29.9 million and \$83.9 million for the three and nine months ended September 30, 2014, respectively. The increase in cost of sales was primarily attributed to the increase in product sales.

## Research and Development

A summary of our various major development programs, including key metrics as of September 30, 2015, is provided below:

Products in Development	Target Indication	Orphan Designation US	Orphan Designation EU	Stage
Kyndrisa	DMD <sup>(1)</sup>	Yes	Yes	Clinical Phase 3
Talazoparib <sup>(2)</sup>	BRCA breast cancer	No	No	Clinical Phase 3
Pegvaliase	PKU	Yes	Yes	Clinical Phase 3
Reveglucosidase alfa	Pompe <sup>(3)</sup>	Yes	Yes	Clinical Phase 2/3
BMN 044 (PRO 044)	DMD <sup>(1)</sup>	Yes	Yes	Clinical Phase 2
BMN 045 (PRO 045)	DMD <sup>(1)</sup>	Yes	Yes	Clinical Phase 2
Vosoritide	Achondroplasia	Yes	Yes	Clinical Phase 2
BMN 053 (PRO 053)	DMD <sup>(1)</sup>	Yes	Yes	Clinical Phase 1/2
Cerliponase alfa	CLN2 <sup>(4)</sup>	Yes	Yes	Clinical Phase 1/2

(1) Duchenne muscular dystrophy, or DMD, acquired from Prosensa in January 2015

(2) Talazoparib is an orally available poly (ADP-ribose) polymerase, or PARP inhibitor for the treatment of patients with certain cancers. In October 2015, we sold this product candidate to Medivation. Under the Asset Purchase Agreement, Medivation will be responsible for all research, development, regulatory and commercialization activities for all indications on a global basis.

(3) Pompe disease, a glycogen storage disorder

(4) CLN2, or late infantile neuronal ceroid lipofuscinosis, is a lysosomal storage disorder primarily affecting the brain.

We manage our R&D expense by identifying the research and development activities we anticipate will be performed during a given period and then prioritizing efforts based on scientific data, probability of successful development, market potential, available human and capital resources and other similar considerations. We continually review our pipeline and the development status of product candidates and, as necessary, reallocate resources among the research and development portfolio that we believe will best support the future growth of our business.

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

R&D expense increased to \$158.7 million for the three months ended September 30, 2015, from \$125.7 million for the three months ended September 30, 2014. R&D expense increased to \$458.7 million for the nine months ended September 30, 2015 from \$319.5 million for the nine months ended September 30, 2014. The increase in R&D expense was primarily a result of the following (in millions):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Change	2015	2014	Change
Vimizim	\$10.5	\$15.3	\$ (4.8 )	\$34.7	\$49.8	\$(15.1 )
Talazoparib <sup>(1)</sup>	17.4	26.0	(8.6 )	55.4	42.7	12.7
Reveglucosidase alfa	15.2	10.9	4.3	45.4	35.2	10.2
Vosoritide	11.2	5.9	5.3	29.8	14.9	14.9
Cerliponase alfa	10.0	11.5	(1.5 )	29.1	25.6	3.5
Pegvaliase	18.6	16.3	2.3	52.3	49.2	3.1
Kyndrisa	14.1	—	14.1	31.5	—	31.5
Mature approved products	10.0	7.5	2.5	26.2	22.3	3.9
Early development stage programs	27.4	15.8	11.6	80.9	36.7	44.2
Not allocated to specific major current projects	24.3	16.5	7.8	73.4	43.1	30.3
<b>Total</b>	<b>\$158.7</b>	<b>\$125.7</b>	<b>\$ 33.0</b>	<b>\$458.7</b>	<b>\$319.5</b>	<b>\$139.2</b>

(1) In October 2015, we sold talazoparib to Medivation. Under the Asset Purchase Agreement, Medivation will be responsible for all research, development, regulatory and commercialization activities for all indications on a global basis.

The increase in talazoparib, reveglucosidase alfa, vosoritide and cerliponase alfa development expense was attributed to increased clinical trial activities related to these product candidates. The development expenses for Kyndrisa relate to clinical and regulatory activities for the product candidate that was acquired with Prosensa in January 2015. The increase in development expense on early development stage programs was primarily attributed to the pre-clinical activity related to BMN 270 and BMN 250. The increase in non-allocated R&D expense was primarily attributed to an increase in R&D personnel costs and facility costs that are not allocated to specific programs. The increase in R&D personnel costs was attributed to an increase in the number of R&D employees and increased stock-based compensation due to the increase in the number of equity awards outstanding and the weighted-average fair value of the equity awards granted in 2015. The increase in facility costs was primarily attributed to non-capitalizable start-up costs for our Shanbally manufacturing facility in preparation for future manufacturing campaigns. Non-allocated R&D expense for the nine months ended September 30, 2014, included a \$6.1 million gain on early lease termination of our San Rafael Corporate Center (SRCC) lease resulting from the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of SRCC. There was no similar gain during the nine months ended September 30, 2015.

During the remainder of 2015, we expect our R&D spending to increase over 2014 levels due to our Kyndrisa, pegvaliase, reveglucosidase alfa, vosoritide and cerliponase alfa programs progressing in their development. Phase 3 clinical trials for pegvaliase and reveglucosidase alfa were initiated in the second quarter of 2013 and in the second quarter of 2014, respectively. We also expect increased spending on pre-clinical and clinical activities for our early development stage programs including, BMN 270, BMN 250 and other pre-clinical programs. Additionally, we expect to continue incurring significant R&D expense for the foreseeable future due to long-term clinical activities related to post-approval regulatory commitments for our approved products. We continuously evaluate the recoverability of costs associated with pre-launch manufacturing activities, and if it is determined that recoverability is highly likely

and therefore future revenues are expected, the costs subsequently incurred related to pre-launch manufacturing activities may be capitalized. When regulatory approval and the likelihood of future revenues for a product candidate are less certain, the related manufacturing costs are expensed as R&D expenses.

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## Selling, General and Administrative

SG&A expense increased to \$94.0 million for the three months ended September 30, 2015, from \$74.6 million for the three months ended September 30, 2014. SG&A expense increased to \$288.4 million for the nine months ended September 30, 2015, from \$202.4 million for the nine months ended September 30, 2014. The increase in SG&A expense was primarily a result of the following (in millions):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Change	2015	2014	Change
Sales and marketing expense (S&M)	\$49.2	\$38.9	\$ 10.3	\$142.0	\$105.1	\$ 36.9
General and administrative expense (G&A)	44.8	35.7	9.1	146.4	97.3	49.1
Total SG&A expense	\$94.0	\$74.6	\$ 19.4	\$288.4	\$202.4	\$ 86.0

S&M expense by product	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Change	2015	2014	Change
Vimizim	\$12.9	\$11.3	\$ 1.6	\$40.5	\$29.7	\$ 10.8
Naglazyme	10.7	12.2	(1.5 )	34.2	37.4	(3.2 )
Kuvan	9.9	8.7	1.2	30.1	25.0	5.1
Other and not allocated	15.7	6.7	9.0	37.2	13.0	24.2
Total S&M expense	\$49.2	\$38.9	\$ 10.3	\$142.0	\$105.1	\$ 36.9

We received regulatory approval to market Vimizim in the U.S. and the EU during 2014 and subsequently in other countries. The increase in Vimizim S&M expense is consistent with the timing of these approvals and its continued world-wide commercial launch. We continue to incur S&M expense for Naglazyme and Kuvan as a result of continued expansion of our international and U.S. activities, respectively. Other S&M expense primarily consists of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. The increase in other S&M expense was driven by an increase in the number of commercial employees and pre-commercialization expense for Kyndrisa and vosoritide.

General and administrative expenses (G&A) primarily consists of corporate support and other administrative expenses, which increased primarily due to increased employee-related expenses as a result of an increase in the number of administrative employees, increased stock based compensation due to the increase in the number of equity awards outstanding and the weighted-average fair value of the equity awards granted in 2015, transaction costs related to the acquisition of Prosensa, consulting fees, and information technology expenses. Corporate support and administrative expenses for the nine months ended September 30, 2014, included a \$2.7 million gain on early lease termination of our SRCC lease resulting from the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of SRCC, which is where our corporate headquarters are located. There was no similar gain during the nine months ended September 30, 2015.

We expect SG&A expense to increase in future periods as a result of the international expansion of Naglazyme and Vimizim, the U.S. commercialization activities for Kuvan, and the increase in administrative support required for our expanding operations.

## Intangible Asset Amortization and Contingent Consideration

Intangible asset amortization and contingent consideration expense is comprised of changes in the fair value of contingent acquisition consideration payable to former stockholders of our acquired businesses, impairment loss (if any) on intangible assets and amortization of intangible assets. Changes in the fair value of contingent acquisition consideration payable result from updates to the estimated probability of achievement or assumed timing of milestones and adjustments to the discount periods and rates. Intangible asset amortization and contingent consideration expense consisted of the following (in millions):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Change	2015	2014	Change
Changes in the fair value of contingent						
acquisition consideration payable	\$0.5	\$1.8	\$ (1.3 )	\$15.1	\$12.6	\$ 2.5
Amortization of intangible assets	0.8	0.8	—	2.4	2.4	—
Total intangible asset amortization and						
contingent consideration	\$1.3	\$2.6	\$ (1.3 )	\$17.5	\$15.0	\$ 2.5

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

The changes in the fair value of the contingent acquisition consideration payable were primarily attributed to changes in the estimated probability of achieving development milestones based on the current status of the related development programs as well as changes in the discount rate utilized in the fair value calculations. During the nine months ended September 30, 2015, the majority of the increase was attributed to development progress of Kyndrisa for which the contingent consideration expense was \$16.2 million.

## Interest Income

We invest our cash, short-term and long-term investments in government and other high credit quality securities in order to limit default and market risk. Interest income totaled \$1.3 million and \$3.1 million for the three and nine months ended September 30, 2015, compared to \$1.4 million and \$4.3 million for the three and nine months ended September 30, 2014. We do not expect future interest income to fluctuate significantly over the next twelve months.

## Interest Expense and Debt Conversion Expense

We incur interest expense on our convertible debt and our capital leases. Interest expense consisted of the following (in millions):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	Change	2015	2014	Change
Coupon interest	\$2.3	\$2.3	\$ —	\$7.7	\$7.1	\$ 0.6
Amortization of issuance costs	0.8	0.8	—	2.5	2.5	—
Accretion of discount on convertible notes	6.3	6.0	0.3	18.7	17.8	0.9
Total interest expense	\$9.4	\$9.1	\$ 0.3	\$28.9	\$27.4	\$ 1.5

Interest expense was primarily comprised of amounts related to our October 2013 issuance of \$750.0 million in aggregate principal amount of senior subordinated convertible debt. We do not expect future interest expense to fluctuate significantly over the next twelve months. See Note 13 to the accompanying Condensed Consolidated Financial Statements for additional information regarding our Convertible Debt.

During the nine months ended September 30, 2015, we recognized Debt Conversion Expense of \$0.2 million in connection with the early conversion of \$8.1 million in aggregate principal amount of our senior subordinated convertible notes due in 2017 (the 2017 Notes). During the nine months ended September 30, 2014, we recognized Debt Conversion expense of \$0.7 million in connection with the early conversion of \$16.5 million in aggregate principal amount of the 2017 Notes.

## Other Income (Expense)

During the second quarter of 2015 we recorded write-offs of \$12.8 million for investments and advances related to a supplier of one of our multi-sourced materials due to a deterioration in their financial condition during the quarter.

## Provision for (Benefit from) Income Taxes

For the three and nine months ended September 30, 2015 we recognized income tax expense of \$0.5 million and an income tax benefit of \$7.3 million, respectively, compared to the three and nine months ended September 30, 2014



when we recognized income tax benefit of \$4.2 million and income tax expense of \$5.0 million, respectively. The provision for (benefit from) income taxes for the three and nine months ended September 30, 2015 and 2014 consisted of state, federal and foreign current tax expense which was offset by deferred tax benefits from federal orphan drug credits and California R&D credits. During the three and nine months ended September 30, 2015, increased R&D expense attributed to an increase in expenses that qualified for the federal orphan drug and California R&D credits as compared to the three and nine months ended September 30, 2014. The provisions for (benefit from) the three and nine months ended September 30, 2015 and 2014 were further reduced by the tax benefit related to stock option exercises during these periods. See Note 15 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2014 for additional discussion of the components of our provision for (benefit from) income taxes.

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## Financial Position, Liquidity and Capital Resources

We expect to fund our operations with our net product revenues from our commercial products, cash, cash equivalents, short-term and long-term investments, supplemented by proceeds from equity or debt financings and loans or collaborative agreements with corporate partners, each to the extent necessary. This expectation could change depending on how much we elect to spend on our development programs, potential licenses and acquisitions of complementary technologies, products and companies or if we elect to settle all or a portion of our convertible debt in cash. We will be highly dependent on our net product revenue to supplement our current liquidity and fund our operations for the foreseeable future. We may in the future elect to supplement this with further debt or equity offerings or commercial borrowing, even after giving effect to our January 2015 equity offering.

We consider the unrepatriated cumulative earnings of certain of our foreign subsidiaries to be indefinitely invested outside the U.S. As of September 30, 2015, \$197.5 million of our \$1,132.7 million balance of cash, cash equivalents and marketable securities was held in foreign subsidiaries, a significant portion of which is required to fund the liquidity needs of these foreign subsidiaries. In managing our liquidity needs in the U.S., we do not rely on the unrepatriated earnings as a source of funds and we have not provided for U.S. federal or state income taxes on these undistributed foreign earnings.

We are mindful that conditions in the current macroeconomic environment could affect our ability to achieve our goals. Some of the factors that could affect our business include: future changes to healthcare reform in the U.S., a continuation of uncertainty with respect to, or worsening of global economic conditions, patent expirations of competitive products and the launch of generic competitors, continued government pricing pressures internationally and the potential volatility in foreign currency exchange rates. We will continue to monitor these conditions and will attempt to adjust our business processes, as appropriate, to mitigate these risks to our business.

Our financial condition as of September 30, 2015 and December 31, 2014 was as follows (in millions):

	September 30, 2015	December 31, 2014	Change
Cash and cash equivalents	\$ 376.3	\$ 875.5	\$(499.2)
Short-term investments	242.0	69.7	172.3
Long-term investments	514.4	97.9	416.5
Cash, cash equivalents and investments	\$ 1,132.7	\$ 1,043.1	\$ 89.6
Current assets	\$ 1,175.6	\$ 1,432.2	\$(256.6)
Current liabilities	390.5	235.7	154.8
Working capital	\$ 785.1	\$ 1,196.5	\$(411.4)
Convertible debt	\$ 667.8	\$ 658.0	\$ 9.8

Our cash flows for each of the nine months ended September 30, 2015 and 2014 are summarized as follows (in millions):

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	2015	2014	Change
Cash and cash equivalents at the beginning of the period	\$875.5	\$568.8	\$306.7
Net cash used in operating activities	(153.7 )	(46.5 )	(107.2)
Net cash used in investing activities	(1,259.1)	(266.4 )	(992.7)
Net cash provided by financing activities	916.2	142.7	773.5
Foreign exchange impact	(2.6 )	(0.6 )	(2.0 )
Cash and cash equivalents at the end of the period	\$376.3	\$398.0	\$(21.7 )
Short-term and long-term investments	756.4	716.7	39.7
Cash, cash equivalents and investments	\$1,132.7	\$1,114.7	\$18.0

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## Working Capital

Working capital decreased by \$411.4 million, from \$1,196.5 million at December 31, 2014 to \$785.1 million at September 30, 2015. The decrease in working capital was attributed to the following (in millions):

Working capital at December 31, 2014	\$1,196.5
Decreased cash, cash equivalents and short-term investments	(326.9 )
Increased accounts receivable, net	4.5
Increased inventory	62.6
Increased current liabilities	(154.8 )
Decreased other current assets	3.2
Working capital at September 30, 2015	\$785.1

The decrease in cash, cash equivalents and short-term investments was primarily attributed to \$538.4 million net cash used to acquire Prosensa, \$153.7 million of cash used to fund operating activities, \$123.8 million net cash invested in property, plant, and equipment, \$12.5 million deposited into an escrow account for the purchase of real property and the net purchase of \$416.5 million of long-term investments, partially offset by net proceeds of \$888.3 million from our January 2015 public offering of common stock and \$29.5 million of net proceeds from employee stock option exercises and employee stock purchase plan (ESPP) contributions. The increase in accounts receivable was attributed to increased revenues and the timing of net product revenues and cash receipts from customers. The increase in inventory was primarily attributed to building of inventories for all commercial products to meet anticipated future sales demand. The increase in current liabilities is primarily comprised of a \$90.4 million increase in short-term contingent acquisition consideration payable to the former Prosensa stockholders, a \$23.4 million increase in accounts payable and accrued operating expenses, a \$17.2 million increase in accrued compensation and a \$12.5 million increase in accrued rebates payable.

Our product sales to government-owned or government-funded customers in certain countries, including Russia, Greece, Spain, Italy and Portugal, are subject to payment terms that are imposed by government authorities. Because these customers are government-owned or government-funded, we may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings, or default in these countries, may decrease the likelihood that we will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to our operating results. Historically we have not experienced a significant level of uncollected receivables and have received continued payments from our more aged accounts. We believe that the allowances for doubtful accounts for these countries are adequate based on our analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. As of September 30, 2015, approximately 8% of our outstanding accounts receivable relate to such countries. See Note 18 to our accompanying Condensed Consolidated Financial Statements for additional discussion. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

## Cash Used in Operating Activities

Cash used in operating activities for the nine months ended September 30, 2015 was \$153.7 million, compared to cash used in operating activities of \$46.5 million for the nine months ended September 30, 2014. Cash used in operating

activities primarily consisted of net loss of \$240.4 million, adjusted for non-cash items such as \$84.5 million for stock-based compensation expenses, \$45.3 million for depreciation and amortization expense, \$14.6 million for deferred income taxes and \$12.8 million for impairment charges. Changes in operating assets and liabilities resulted in a net cash outflow of \$89.5 million that consisted primarily of increased inventories for all commercial products to meet anticipated future sales demand, prepaid taxes and restricted investments, which secure our irrevocable standby letter of credit obtained in connection with certain commercial agreements, offset by a decrease in the carrying value of forward foreign exchange contracts due to settlements during the period.

Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Cash Used in Investing Activities

Net cash used in investing activities during the nine months ended September 30, 2015 and 2014 was \$1,259.1 million and \$266.4 million, respectively. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures, such as manufacturing equipment and facility construction and improvements. The increase in net cash used by investing activities for the nine months ended September 30, 2015 compared to the nine months ended September 30, 2014 was primarily comprised of a \$538.4 million increase due to the Prosensa acquisition, net of cash acquired, a \$47.0 million increase in capital expenditures and an increase of \$339.6 million in net purchases of investments. During the remainder of 2015, we expect to make significant capital investments in our Shanbally, Ireland manufacturing facility to enable future commercial manufacturing of our products at the facility and our corporate headquarters at SRCC to accommodate additional laboratory space requirements and anticipated headcount growth.

Cash Provided by Financing Activities

Net cash provided by financing activities for the nine months ended September 30, 2015 was \$916.2 million, compared to net cash provided by financing activities of \$142.7 million for the nine months ended September 30, 2014. Historically, our financing activities primarily included proceeds from the sale of our convertible debt, common stock and employee stock purchases under the ESPP and employee stock option exercises, offset by payments related to our contingent acquisition obligations and convertible debt obligations. The increase in net cash provided by financing activities for the nine months ended September 30, 2015 was primarily attributed to a \$770.8 million increase in net proceeds from our January 2015 equity offering compared to the March 2014 equity offering.

Other Information

On January 15, 2015, we closed the initial offering period related to our offer to purchase all of the ordinary shares (Prosensa Shares) of Prosensa Holding N.V. (Prosensa), a public limited liability company organized under the laws of the Netherlands, purchasing 93.4% of the Prosensa Shares and immediately launched a subsequent offering period that expired on January 29, 2015. As of the expiration of the subsequent offering period, we paid approximately \$620.7 million for approximately 35 million Prosensa Shares, representing approximately 96.8% of all the outstanding Prosensa Shares. Additionally, we paid approximately \$38.6 million for the options that vested pursuant to the definitive purchase agreement. On February 12, 2015, we completed an asset transfer and we paid \$20.8 million to the remaining Prosensa shareholders. Effective February 12, 2015, Prosensa has been dissolved and is in liquidation under Dutch law.

On January 27, 2015, we sold approximately 9.8 million shares of our common stock at a price of \$93.25 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds of approximately \$888.3 million from this public offering after underwriter's discount and offering costs.

On October 1, 2015 we entered into a definitive agreement to acquire all global rights to Kuvan and pegvaliase from Merck Serono, with the exception of Kuvan in Japan. Under the terms of the agreement, we will provide Merck with an upfront payment of €340.0 million. We have also agreed to pay Merck Serono additional consideration in future periods up to €185.0 million (undiscounted) in milestones payments if certain pegvaliase regulatory and sales milestones are attained. Previously, we had exclusive rights to Kuvan in the U.S. and Canada and to pegvaliase in the U.S. and Japan. Under the terms of the agreement, we will now have exclusive worldwide rights to Kuvan and pegvaliase with the exception of Kuvan in Japan. The closing of the Merck Serono transaction is subject to the

customary closing conditions, including regulatory approval, and we expect to close the transaction in January 2016.

On October 6, 2015, we completed the sale of talazoparib to Medivation, Inc. (Medivation), under which Medivation acquired the worldwide rights to talazoparib in exchange for an upfront payment of \$410.0 million and up to an additional \$160.0 million upon the achievement of regulatory and sales-based milestones and mid-single digit percentage royalties for talazoparib.

Our \$781.6 million (undiscounted) of total convertible debt as of September 30, 2015 will impact our liquidity due to the semi-annual cash interest payments and will further impact our liquidity if we elect to settle all or portions of the 2018 Notes or the 2020 Notes in cash upon conversion or if the holders of our 2017 Notes do not convert on or prior to the scheduled repayments of the debt. Further, depending on market conditions, our financial position and performance and other factors, we may in the future choose to use a portion of our cash or cash equivalents to repurchase our convertible debt or other securities.

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## Funding Commitments

We cannot estimate with certainty the cost to complete any of our product development programs. Additionally, except as disclosed under “Overview” above, we cannot precisely estimate the time to complete any of our product development programs or when we expect to receive net cash inflows from any of our product development programs. Please see “Risk Factors” included in Part II, Item 1A of this Quarterly Report on Form 10-Q, for a discussion of the reasons we are unable to estimate such information, and in particular the following risk factors:

- If we fail to obtain or maintain regulatory approval to commercially market and sell our drugs, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased;
- If we are unable to successfully develop and maintain manufacturing processes for our drug products to produce sufficient quantities at acceptable costs, we may be unable to meet demand for our products and lose potential revenue, have reduced margins or be forced to terminate a program;
- If we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenue could be adversely affected; and
- If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

Our investment in our product development programs and continued development of our existing commercial products has a major impact on our operating performance. Our R&D expenses in the period since inception of our major programs were as follows (in millions):

	Since Program Inception
Vimizim	\$ 392.1
Talazoparib <sup>(1)</sup>	171.8
Reveglucosidase alfa	193.7
Vosoritide	99.2
Cerliponase alfa	100.2
Pegvaliase	290.5
Kyndrisa	31.5
Mature approved products	429.5
Not allocated to specific major current projects	Not meaningful

(1) In October 2015, we sold talazoparib to Medivation. Under the Asset Purchase Agreement, Medivation will be responsible for all research, development, regulatory and commercialization activities for all indications on a global basis.

We may elect to increase our spending above our current long-term plans and consequently we may be unable to achieve our long-term goals. This may increase our capital requirements, including: costs associated with the commercialization of our products; additional clinical trials; investments in the manufacturing of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse; preclinical studies and clinical trials for our other product candidates; potential licenses and other acquisitions of complementary technologies, products and companies; and general corporate purposes.



Our future capital requirements will depend on many factors, including, but not limited to:

- product sales and profitability of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- manufacturing, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- progress of our integration of Prosensa;
- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

- results relating to our lawsuit against DRL and Par to protect our patents relating to Kuvan;
- government regulatory action affecting our product candidates or our competitors' drug products in both the U.S. and non-U.S. countries;
- developments or disputes concerning patent or proprietary rights;
- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;
- economic conditions in the U.S. or abroad;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results; and
- changes in company assessments or financial estimates by securities analysts.

## Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements that are currently material or reasonably likely to be material to our consolidated financial position or results of operations.

## Contractual and Commercial Obligations

We have contractual and commercial obligations under our debt, operating leases and other obligations related to R&D activities, purchase commitments, licenses and sales royalties with annual minimums. Our contractual obligations for non-cancelable purchase commitments as of September 30, 2015 are presented in the table below (in millions).

	Payments Due within				Total
	1 Year or Less	>1 -3 Years	> 3 - 5 Years	More Than 5 Years	
2017 Notes and related interest	\$0.3	\$32.5	\$—	\$—	\$32.8
2018 Notes and related interest	1.4	5.6	377.8	—	384.8
2020 Notes and related interest	2.8	11.2	11.2	380.6	405.8
Operating leases	2.1	19.4	15.5	0.1	37.1
R&D and purchase commitments	40.1	16.6	2.2	—	58.9
Total	\$46.7	\$85.3	\$406.7	\$380.7	\$919.4

We are also subject to contingent payments totaling approximately \$763.0 million as of September 30, 2015 which are due upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, \$160.0 million relates to the acquisition of Prosensa and \$22.9 million relates to programs that are no longer being developed.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Our market risks during the nine months ended September 30, 2015 have not materially changed from those discussed in Part II, Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2014, which was filed with the SEC on March 2, 2015.

Item 4. Controls and Procedures

(a) Controls and Procedures

An evaluation was carried out, under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness 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 (the Exchange Act), as of the end of the period covered by this report.

Based on the evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures are effective to ensure that the information required to be disclosed by us in the reports we file or submit under the Exchange Act was recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms.

(b) Change in Internal Controls over Financial Reporting

There were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act, during our most recently completed quarter that have materially affected or are reasonably likely to materially affect our internal control over financial reporting. We are utilizing the Committee of Sponsoring Organizations of the Treadway Commission (COSO) 2013 Framework on internal control.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings.

Paragraph IV Notices

As previously disclosed, we received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL has filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the U.S. Food and Drug Administration's (the FDA) Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie, we filed lawsuits against both DRL and Par in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan triggering the automatic 30-month stay on the approval of each ANDA. In response, DRL and Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

In September 2015, we entered into a settlement agreement with DRL that resolved patent litigation with DRL in the United States related to Kuvan (sapropterin dihydrochloride) 100 mg oral tablets. The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending. The Court has not yet set a date for a claim construction hearing or trial in the litigation against Par.

#### Item 1A. Risk Factors

An investment in our securities involves a high degree of risk. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the value of our securities to decline, and you may lose all or part of your investment.

We have marked with an asterisk (\*) those risk factors below that include a substantive change from or update to the risk factors included in our Annual Report on Form 10-K, for the year ended December 31, 2014, which was filed with the SEC on March 2, 2015.

## Risks Related to Our Business

\*If we fail to obtain or maintain regulatory approval to commercially market and sell our drugs, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.

We must obtain and maintain regulatory approval to market and sell our drug products in the U.S. and in jurisdictions outside of the U.S. In the U.S., we must obtain FDA approval for each drug that we intend to commercialize. The FDA approval process is typically lengthy and expensive, and approval is never certain. Products distributed abroad are also subject to government regulation by international regulatory authorities. The approval process in the EU and other countries can also be lengthy and expensive and regulatory approval is also never certain. Naglazyme, Aldurazyme, Kuvan and Vimizim have received regulatory approval to be commercially marketed and sold in the U.S., the EU and other countries. Firdapse has received regulatory approval to be commercially marketed only in the EU.

As part of the recent reauthorization of the Prescription Drug User Fee Act, new biologics are included in a new product review program intended to enhance FDA-sponsor communications to lead to greater first-cycle approval decisions. As part of this program, applications for new biologics are subject to either a 12-month standard or 8-month priority review period that begins from the date of application submission. However, since this is a new product review program and few products have completed this new review process, the priority review period may take longer than eight months and the standard review period may take longer than 12 months. Similarly, although the EMA has an accelerated approval process, the timelines mandated by the regulations are subject to the possibility of substantial delays.

In addition, the FDA and its international equivalents have substantial discretion over the approval process for pharmaceutical products. As such, these regulatory agencies may in the end not agree that we have demonstrated the requisite level of product safety and efficacy to grant approval and may require additional data. If we fail to obtain regulatory approval for our product candidates, we will be unable to market and sell those drug products. Because of the risks and uncertainties in pharmaceutical development, our product candidates could take a significantly longer time to gain regulatory approval than we expect or may never gain approval. We also rely on independent third-party contract research organizations (CROs) to file some of our ex-U.S. and ex-EU marketing applications and important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, if the CRO elects to prioritize work on our projects below other projects or if there is any dispute or disruption in our relationship with our CROs, the filing of our applications may be delayed.

From time to time during the regulatory approval process for our products and our product candidates, we engage in discussions with the FDA and comparable international regulatory authorities regarding the regulatory requirements for our development programs. To the extent appropriate, we accommodate the requests of the regulatory authorities and, to date, we have generally been able to reach reasonable accommodations and resolutions regarding the underlying issues. However, we are often unable to determine the outcome of such deliberations until they are final. If we are unable to effectively and efficiently resolve and comply with the inquiries and requests of the FDA and other non-U.S. regulatory authorities, the approval of our product candidates may be delayed and their value may be reduced.

After any of our products receive regulatory approval, they remain subject to ongoing regulation, which can impact, among other things, product labeling, manufacturing practices, adverse event reporting, storage, expiration, distribution, advertising and promotion, record keeping and import and export. If we do not comply with the applicable regulations, the range of possible sanctions includes issuance of warning or untitled letters or adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of

marketing applications, and other enforcement actions, including injunctions and civil or criminal prosecution. The FDA and comparable international regulatory agencies can withdraw a product's approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues. Further, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved products. The FDA and comparable international regulatory agencies may condition approval of our product candidates on the completion of such post-marketing clinical studies. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient. If data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is delayed or withdrawn, the value of our company and our operating results will be adversely affected. Additionally, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased.

Some of our product candidates, including cerliponase alfa, are intended to be used in combination with a delivery device, such as an injector or other delivery system. Medical products containing a combination of new drugs, biological products or medical devices may be regulated as “combination products” in the United States. A combination product generally is defined as a product comprised of components from two or more regulatory categories (e.g., drug/device, device/biologic, drug/biologic). Each component of a combination product is subject to the requirements established by the FDA for that type of component, whether a new drug, biologic or device. In order to facilitate pre-market review of combination products, the FDA designates one of its centers to have primary jurisdiction for the pre-market review and regulation of the overall product based upon a determination by the FDA of the primary mode of action of the combination product. The determination whether a product is a combination product or two separate products is made by the FDA on a case-by-case basis. Our product candidates intended for use with such devices, or expanded indications that we may seek for our products used with such devices, may not be approved or may be substantially delayed in receiving approval if the devices do not gain and/or maintain their own regulatory approvals or clearances. Where approval of the drug or biologic product and device is sought under a single application, the increased complexity of the review process may delay approval. The FDA review process and criteria is not a well-established area, which could also lead to delays in the approval process. In addition, because these delivery devices are provided by unaffiliated third-party companies, we are dependent on the sustained cooperation and effort of those third-party companies both to obtain regulatory approval and to maintain their own regulatory compliance. Failure of third-party companies to assist in the approval process or to maintain their own regulatory compliance could delay or prevent approval of our product candidates, or limit our ability to sell a product once approved.

If we fail to obtain or maintain orphan drug exclusivity for some of our products, our competitors may sell products to treat the same conditions and our revenues will be reduced.

As part of our business strategy, we intend to develop some drugs that may be eligible for FDA and EU orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the U.S. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. Similar regulations are available in the EU with a ten-year period of market exclusivity.

Because the extent and scope of patent protection for some of our drug products is limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the exclusivity period under the Orphan Drug Act to maintain a competitive position. If we do not obtain orphan drug exclusivity for our drug products that do not have broad patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced.

Even though we have obtained orphan drug designation for certain of our products and product candidates and even if we obtain orphan drug designation for our future product candidates, due to the uncertainties associated with developing biopharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication, which means that we may not obtain orphan drug exclusivity and could also potentially be blocked from approval until the first product’s orphan drug exclusivity period expires. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved and granted orphan drug exclusivity, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.





We may face competition from biological products approved through an abbreviated regulatory pathway.

Our Naglazyme, Aldurazyme and Vimizim products are regulated by the FDA as biologics under the Federal Food, Drug, and Cosmetic Act (the FDC Act) and the Public Health Service Act (the PHS Act). Biologics require the submission of a biologics license application (BLA) and approval by the FDA prior to being marketed in the U.S. Historically, a biologic product approved under a BLA was not subject to the generic drug review and approval provisions of the FDC Act. However, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (as amended, the PPACA) created a regulatory pathway under the PHS Act for the abbreviated approval for biological products that are demonstrated to be “biosimilar” or “interchangeable” with an FDA-approved biological product. In order to meet the standard of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. Such biosimilars would reference biological products approved in the U.S. The law establishes a period of 12 years of data exclusivity for reference products, which protects the data in the original BLA by prohibiting sponsors of biosimilars from gaining FDA approval based in part on reference to data in the original BLA. Our products approved under BLAs, as well as products in development that may be approved under BLAs, could be reference products for such biosimilar marketing applications.

To obtain regulatory approval to market our products, preclinical studies and costly and lengthy preclinical and clinical trials are required and the results of the studies and trials are highly uncertain.

As part of the regulatory approval process we must conduct, at our own expense, preclinical studies in the laboratory and clinical trials on humans for each product candidate. We expect the number of preclinical studies and clinical trials that the regulatory authorities will require will vary depending on the product candidate, the disease or condition the drug is being developed to address and regulations applicable to the particular drug. Generally, the number and size of clinical trials required for approval increase based on the expected patient population that may be treated with a drug. We may need to perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays in our ability to market any of our product candidates. Furthermore, even if we obtain favorable results in preclinical studies, the results in humans may be significantly different. After we have conducted preclinical studies, we must demonstrate that our drug products are safe and efficacious for use in the targeted human patients in order to receive regulatory approval for commercial sale. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, and favorable data from interim analyses do not ensure the final results of a trial will be favorable. Product candidates may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials, or despite having favorable data in connection with an interim analysis. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Our future clinical trial results may not be successful.

Adverse or inconclusive clinical results would stop us from filing for regulatory approval of our product candidates. Additional factors that can cause delay or termination of our clinical trials include:

- slow or insufficient patient enrollment;
- slow recruitment of, and completion of necessary institutional approvals at, clinical sites;
- longer treatment time required to demonstrate efficacy;
- lack of sufficient supplies of the product candidate;
- adverse medical events or side effects in treated patients;

- lack of effectiveness of the product candidate being tested; and
- regulatory requests for additional clinical trials or pre-clinical studies.

Typically, if a drug product is intended to treat a chronic disease, as is the case with some of our product candidates, safety and efficacy data must be gathered over an extended period of time, which can range from nine months to three years or more. We also rely on independent third-party CROs to perform most of our clinical studies and many important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, or if there is any dispute or disruption in our relationship with our CROs, our clinical trials may be delayed. Moreover, in our regulatory submissions, we rely on the quality and validity of the clinical work performed by third-party CROs. If any of our CROs' processes, methodologies or results were determined to be not conducted in accordance with current good clinical practices, invalid or inadequate, our own clinical data and results and related regulatory approvals could adversely be impacted.

If we continue to incur operating losses for a period longer than anticipated, we may be unable to continue our operations at planned levels and be forced to reduce our operations.

Since we began operations in March 1997, we have been engaged in substantial research and development and operated at a net loss until 2008. Although we were profitable in 2008, 2010 and the third quarter of 2014, we operated at a net loss in 2009, 2011, 2012, 2013 and 2014. Based upon our current plan for investments in research and development for existing and new programs, we expect to operate at a net loss for at least the next 12 months. Our future profitability depends on our marketing and selling of Vimizim, Naglazyme, Kuvan and Firdapse, the successful continued commercialization of Aldurazyme by Genzyme, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any approved drugs, either by ourselves or jointly with others, our spending on our development programs and the impact of any possible future business development transactions. The extent of our future losses and the timing of profitability are highly uncertain. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

If we fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.

Before we can begin commercial manufacture of our products, regulatory authorities must approve marketing applications that identify manufacturing facilities operated by us or our contract manufacturers that have passed regulatory inspection and manufacturing processes that are acceptable to the regulatory authorities. In addition, our pharmaceutical manufacturing facilities are continuously subject to inspection by the FDA and international regulatory authorities, before and after product approval. Our manufacturing facilities in the U.S. have been approved by the FDA, the European Commission (the EC), and health agencies in other countries for the manufacture of Aldurazyme and Naglazyme. In addition, our third-party manufacturers' facilities involved with the manufacture of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse have also been inspected and approved by various regulatory authorities. Although we are not involved in the day-to-day operations of our contract manufacturers, we are ultimately responsible for ensuring that our products are manufactured in accordance with current Good Manufacturing Practices (cGMP) regulations. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011 has not yet been approved by the FDA or the EMA to manufacture any of our products. We intend to make a substantial investment in the build-out of the Shanbally facility in order to manufacture Vimizim and other products. If the facility is not ultimately approved by the FDA or the EMA to manufacture any of our products, we will not be able to manufacture Vimizim or other products at this facility and we may not be able to meet the anticipated commercial demand for Vimizim which would have an adverse effect on our financial results.

Due to the complexity of the processes used to manufacture our products and product candidates, we may be unable to continue to pass or initially pass federal or international regulatory inspections in a cost-effective manner. For the same reason, any potential third-party manufacturer of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse or our product candidates may be unable to comply with cGMP regulations in a cost-effective manner and may be unable to initially or continue to pass a federal or international regulatory inspection.

If we, or third-party manufacturers with whom we contract, are unable to comply with manufacturing regulations, we may be subject to delay of approval of our product candidates, warning or untitled letters, fines, unanticipated compliance expenses, recall or seizure of our products, total or partial suspension of production and/or enforcement actions, including injunctions, and criminal or civil prosecution. These possible sanctions would adversely affect our financial results and financial condition.

\*If we fail to obtain the capital necessary to fund our operations, our financial results and financial condition will be adversely affected and we will have to delay or terminate some or all of our product development programs.

As of September 30, 2015, we had cash, cash equivalents and short and long-term investments totaling \$1,132.7 million and long-term debt obligations of \$781.6 million (undiscounted). In January 2015, we paid \$620.7 million for approximately 35 million Prosensa Shares, representing approximately 96.8% of all outstanding Prosensa Shares, and \$38.6 million for the options that vested pursuant to the definitive purchase agreement. In February 2015, we completed the Prosensa asset transfer and paid \$20.8 million to the remaining Prosensa shareholders. We (through our indirect wholly-owned subsidiaries) funded the acquisition with our available cash balances. We expect to pay up to \$160.0 million if certain development milestones are attained. In October 2013, we completed an offering of senior subordinated convertible notes and received net proceeds of approximately \$696.4 million, after deducting commissions, estimated offering expenses payable by us and the purchase of the related capped calls. We will need cash to not only repay the principal amount of our 0.75% senior subordinated convertible notes due 2018 and 1.50% senior subordinated convertible notes due in 2020 (collectively, the Notes) but also the ongoing interest due on the Notes during their term. In March 2014, we completed an offering of 1,500,000 shares of our common stock at a price of \$78.45 per share and received net proceeds of \$117.5 million. In January 2015, we completed an offering of 9,775,000 shares of our common stock at a price of \$93.25 per share and received net proceeds of approximately \$888.3 million. We may require additional financing to fund our future operations, including the commercialization of our approved drugs and drug product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing, if needed, due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise any necessary additional financing we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

We expect to continue to spend substantial amounts of capital for our operations for the foreseeable future. The amount of capital we will need depends on many factors, including:

- our ability to successfully market and sell Vimizim, Naglazyme, Kuvan and Firdapse;
  - Genzyme's ability to continue to successfully commercialize Aldurazyme;
- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the progress of research programs carried out by us;
- our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD Therapeutics, Inc., ZyStor, Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc. and under the CVRs issued in connection with the acquisition of Prosensa that trigger related milestone payments;
- any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish; and
- whether our convertible debt is converted to common stock in the future.

Moreover, our fixed expenses such as rent, license payments, interest expense and other contractual commitments are substantial and may increase in the future. These fixed expenses may increase because we may enter into:

- additional licenses and collaborative agreements;
- additional contracts for product manufacturing; and

·additional financing facilities.

We may need to raise additional funds from equity or debt securities, loans or collaborative agreements if we are unable to satisfy our liquidity requirements. The sale of additional securities may result in additional dilution to our stockholders. Furthermore, additional financing may not be available in amounts or on terms satisfactory to us or at all. This could result in the delay, reduction or termination of our research, which could harm our business.

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\*If we are unable to successfully develop and maintain manufacturing processes for our drug products to produce sufficient quantities at acceptable costs, we may be unable to meet demand for our products and lose potential revenue, have reduced margins or be forced to terminate a program.

Due to the complexity of manufacturing our products, we may not be able to manufacture drug products successfully with a commercially viable process or at a scale large enough to support their respective commercial markets or at acceptable margins.

The development of commercially viable manufacturing processes typically is very difficult to achieve and is often very expensive and may require extended periods of time. Changes in manufacturing processes (including manufacturing cell lines), equipment or facilities may require us to complete clinical trials to receive regulatory approval of any manufacturing improvements. Also, we may be required to demonstrate product comparability between a biological product made after a manufacturing change and the product made before implementation of the change through additional types of analytical and functional testing or may have to complete additional clinical studies. If we contract for manufacturing services with an unproven process, our contractor is subject to the same uncertainties, high standards and regulatory controls, and may therefore experience difficulty if further process development is necessary.

Even a developed manufacturing process can encounter difficulties. Problems may arise during manufacturing for a variety of reasons, including human error, mechanical breakdowns, problems with raw materials and cell banks, malfunctions of internal information technology systems, and other events that cannot always be prevented or anticipated. Many of the processes include biological systems, which add significant complexity, as compared to chemical synthesis. We expect that, from time to time, consistent with biotechnology industry expectations, certain production lots will fail to produce product that meets our quality control release acceptance criteria. To date, our historical failure rates for all of our product programs, including Naglazyme, Aldurazyme and Vimizim, have been within our expectations, which are based on industry norms. If the failure rate increased substantially, we could experience increased costs, lost revenue, damage to customer relations, time and expense investigating the cause and, depending upon the cause, similar losses with respect to other lots or products. If problems are not discovered before the product is released to the market, recall and product liability costs may also be incurred.

In order to produce product within our time and cost parameters, we must continue to produce product within our expected success rate and yield expectations. Because of the complexity of our manufacturing processes, it may be difficult or impossible for us to determine the cause of any particular lot failure and we must effectively take corrective action in response to any failure in a timely manner.

Although we have entered into contractual relationships with third-party manufacturers to produce the active ingredient in Kuvan, Firdapse and Kyndrisa (formerly referred to as drisapersen), if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. We have contracts for the production of final product for Kuvan, Firdapse and Kyndrisa. We also rely on third-parties for portions of the manufacture of Naglazyme, Aldurazyme and Vimizim. If those manufacturers are unwilling or unable to fulfill their contractual obligations or satisfy demand outside of or in excess of the contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. Further, the availability of suitable contract manufacturing capacity at scheduled or optimum times is not certain.

In addition, our manufacturing processes subject us to a variety of federal, state and local laws and regulations governing the use, generation, manufacture, storage, handling and disposal of hazardous materials and wastes resulting from their use. We may incur significant costs in complying with these laws and regulations.

If we are unable to effectively address manufacturing issues, we may be unable to meet demand for our products and lose potential revenue, have reduced margins, or be forced to terminate a program.

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Our manufacturing facility for Naglazyme, Aldurazyme and Vimizim is located near known earthquake fault zones, and the occurrence of an earthquake or other catastrophic disaster could cause damage to our facility and equipment, or that of our third-party manufacturers or single-source suppliers, which could materially impair our ability to manufacture Naglazyme, Aldurazyme and Vimizim or our third-party manufacturer's ability to manufacture Kuvan or Firdapse.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Naglazyme, Aldurazyme and Vimizim. It is located in the San Francisco Bay Area near known earthquake fault zones and is vulnerable to significant damage from earthquakes. We, the third-party manufacturers with whom we contract and our single-source suppliers of raw materials, which include many of our critical raw materials, are also vulnerable to damage from other types of disasters, including fires, floods, power loss and similar events. If any disaster were to occur, or any terrorist or criminal activity caused significant damage to our facilities or the facilities of our third-party manufacturers and suppliers, our ability to manufacture Naglazyme, Aldurazyme and Vimizim, or to have Kuvan or Firdapse manufactured, could be seriously, or potentially completely impaired, and our commercialization efforts and revenue could be seriously impaired. The insurance that we carry, the inventory that we maintain and our risk mitigation plans may not be adequate to cover our losses resulting from disasters or other business interruptions.

Supply interruptions may disrupt our inventory levels and the availability of our products and product candidates and cause delays in obtaining regulatory approval for our product candidates, or harm our business by reducing our revenues.

Numerous factors could cause interruptions in the supply of our products and product candidates, including:

- timing, scheduling and prioritization of production by our contract manufacturers or a breach of our agreements by our contract manufacturers;
- labor interruptions;
- changes in our sources for manufacturing;
- the timing and delivery of shipments;
- our failure to locate and obtain replacement manufacturers as needed on a timely basis; and
- conditions affecting the cost and availability of raw materials.

Any interruption in the supply of finished products could hinder our ability to distribute finished products to meet commercial demand.

With respect to our product candidates, production of product is necessary to perform clinical trials and successful registration batches are necessary to file for approval to commercially market and sell product candidates. Delays in obtaining clinical material or registration batches could adversely impact our clinical trials and delay regulatory approval for our product candidates.

Because the target patient populations for our products are small, we must achieve significant market share and maintain high per-patient prices for our products to achieve profitability.

All of our products target diseases with small patient populations. As a result, our per-patient prices must be relatively high in order to recover our development and manufacturing costs and achieve profitability. For Naglazyme and Vimizim we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in the disease populations are small, it is not only important to find patients who begin therapy to achieve significant market penetration of the product, but we also need to be able to maintain these patients on therapy for an extended period of time. Due to the expected costs of treatment for our products for genetic diseases, we may be unable to maintain or obtain sufficient market share at a price high enough to justify our product development efforts and manufacturing expenses.



If we fail to obtain an adequate level of coverage and reimbursement for our drug products by third-party payers, the sales of our drugs would be adversely affected or there may be no commercially viable markets for our products.

The course of treatment for patients using our products is expensive. We expect patients to need treatment for extended periods, and for some products throughout the lifetimes of the patients. We expect that most families of patients will not be capable of paying for this treatment themselves. There will be no commercially viable market for our products without coverage and reimbursement from third-party payers. Additionally, even if there is a commercially viable market, if the level of reimbursement is below our expectations, our revenue and gross margins will be adversely affected.

Third-party payers, such as government or private health care insurers, carefully review and increasingly challenge the prices charged for drugs. Reimbursement rates from private companies vary depending on the third-party payer, the insurance plan and other factors. Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis.

Reimbursement in the EU and many other territories must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until reimbursement is approved. The timing to complete the negotiation process in each country is highly uncertain, and in some countries we expect that it may exceed 12 months. Even after a price is negotiated, countries frequently request or require adjustments to the price and other concessions over time.

For our future products, we will not know what the reimbursement rates will be until we are ready to market the product and we actually negotiate the rates. If we are unable to obtain sufficiently high reimbursement rates for our products, they may not be commercially viable or our future revenues and gross margins may be adversely affected.

A significant portion of our international sales are made based on special access programs, and changes to these programs could adversely affect our product sales and revenue in these countries.

We make a significant portion of our international sales of Naglazyme and Vimizim through special access or “named patient” programs, which do not require full product approval. The specifics of the programs vary from country to country. Generally, special approval must be obtained for each patient. The approval normally requires an application or a lawsuit accompanied by evidence of medical need. Generally, the approvals for each patient must be renewed from time to time.

These programs are not well defined in some countries and are subject to changes in requirements and funding levels. Any change to these programs could adversely affect our ability to sell our products in those countries and delay sales. If the programs are not funded by the respective government, there could be insufficient funds to pay for all patients. Further, governments have in the past undertaken and may in the future undertake, unofficial measures to limit purchases of our products, including initially denying coverage for purchasers, delaying orders and denying or taking excessively long to approve customs clearance. Any such actions could materially delay or reduce our revenues from such countries.

Without the special access programs, we would need to seek full product approval to commercially market and sell our products. This can be an expensive and time-consuming process and may subject our products to additional price controls. Because the number of patients is so small in some countries, it may not be economically feasible to seek and maintain a full product approval, and therefore the sales in such country would be permanently reduced or eliminated. For all of these reasons, if the special access programs that we are currently using are eliminated or restricted, our revenues could be adversely affected.

If we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenue could be adversely affected.

Our competitors may develop, manufacture and market products that are more effective or less expensive than ours. They may also obtain regulatory approvals for their products faster than we can obtain them (including those products with orphan drug designation) or commercialize their products before we do. If we do not compete successfully, our revenue would be adversely affected, and we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement may be increasingly restricted both in the U.S. and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. Governmental and private third-party payers have proposed health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, including the cost of drug treatments, have been made in the U.S. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding health care may affect coverage and reimbursement for medical treatment by third-party payers, which may render our products not commercially viable or may adversely affect our future revenues and gross margins.

International operations are also generally subject to extensive price and market regulations, and there are many proposals for additional cost-containment measures, including proposals that would directly or indirectly impose additional price controls or mandatory price cuts or reduce the value of our intellectual property portfolio. As part of these cost containment measures, some countries have imposed or threatened to impose revenue caps limiting the annual volume of sales of Naglazyme. To the extent that these caps are significantly below actual demand, our future revenues and gross margins may be adversely affected.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

Government health care reform could increase our costs, and would adversely affect our revenue and results of operations.

Our industry is highly regulated and changes in law may adversely impact our business, operations or financial results. The PPACA is a sweeping measure intended to expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program.

Several provisions of the law, which have varying effective dates, may affect us and will likely increase certain of our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. Among other things, the PPACA also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance; included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or “donut hole,” and imposed a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The law also revised the definition of “average manufacturer price” for reporting purposes, which could increase the amount of the Medicaid drug rebates paid to states.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and will remain in effect through 2024 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

We anticipate that the PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and an additional downward pressure on the reimbursement our customers may receive for our products. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

We face credit risks from customers outside of the U.S. that may adversely affect our results of operations.

Our product sales to government-owned or supported customers in various countries outside of the U.S. are subject to significant payment delays due to government funding and reimbursement practices. This has resulted and may continue to result in an increase in days sales outstanding due to the average length of time that we have accounts

receivable outstanding. If significant changes were to occur in the reimbursement practices of these governments or if government funding becomes unavailable, we may not be able to collect on amounts due to us from these customers and our results of operations would be adversely affected.

If we are found in violation of federal or state “fraud and abuse” laws, we may be required to pay a penalty or be suspended from participation in federal or state health care programs, which may adversely affect our business, financial condition and results of operation.

We are subject to various federal and state health care fraud and abuse laws, including anti-kickback laws, false claims laws and laws related to ensuring compliance. The federal health care program anti-kickback statute makes it illegal for any person, including a pharmaceutical company, to knowingly and willfully offer, solicit, pay or receive any remuneration, directly or indirectly, in exchange for or to induce the referral of business, including the purchase, order or prescription of a particular drug, for which payment may be made under federal health care programs, such as Medicare and Medicaid. Under federal government regulations, certain arrangements, or safe harbors, are deemed not to violate the federal anti-kickback statute. However, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration not intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from anti-kickback liability, although we seek to comply with these safe harbors. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs.

Federal and state false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Under the Health Insurance Portability and Accountability Act of 1996, we also are prohibited from knowingly and willfully executing a scheme to defraud any health care benefit program, including private payers, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a manufacturer’s products from reimbursement under government programs, criminal fines and imprisonment.

Many states have adopted laws similar to the federal anti-kickback statute, some of which apply to referral of patients for health care services reimbursed by any source, not just governmental payers.

Substantial new provisions affecting compliance have also been adopted, which may require us to modify our business practices with health care practitioners. The PPACA, among other things, requires drug manufacturers to collect and report information on payments or transfers of value to physicians and teaching hospitals, as well as investment and ownership interests held by physicians and their immediate family members during the preceding calendar year. Failure to submit required information may result in civil monetary penalties. Manufacturers were required to begin collecting required information on August 1, 2013 and the Centers for Medicare & Medicaid Services (CMS) made public the reported data in a searchable form on September 30, 2014. Manufacturers are required to submit reports to CMS by the 90<sup>th</sup> day of each subsequent calendar year.

In addition, there has been a recent trend of increased state regulation of payments made to physicians. Certain states mandate implementation of compliance programs, compliance with the Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the PhRMA Code on Interactions with Healthcare Professionals, and/or the tracking and reporting of gifts, compensation and other remuneration to physicians. The shifting compliance environment and the need to implement systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a pharmaceutical manufacturer may violate one or more of the requirements.

While we believe we have structured our business arrangements to comply with these laws, because of the breadth of these laws, the narrowness of available statutory and regulatory exceptions and the increased focus by law

enforcement agencies in enforcing such laws, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has strengthened, these laws. For example, the PPACA, among other things, amends the intent requirement of the federal anti-kickback and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. Moreover, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the False Claims Act. If we are found in violation of one of these laws, we may be subject to criminal, civil or administrative sanctions, including debarment, suspension or exclusion from participation in federal or state health care programs any of which could adversely affect our business, financial condition and results of operation.

We conduct a significant amount of our sales and operations outside of the U.S., which subjects us to additional business risks that could adversely affect our revenue and results of operations.

A significant portion of the sales of Aldurazyme, Naglazyme and Vimizim, and all of the sales of Firdapse are generated from countries other than the U.S. Additionally, we have operations in several European countries, Brazil, other Latin American countries, Turkey and other Asian countries. We expect that we will continue to expand our international operations in the future. International operations inherently subject us to a number of risks and uncertainties, including:

- changes in international regulatory and compliance requirements that could restrict our ability to manufacture, market and sell our products;
- political and economic instability;
- diminished protection of intellectual property in some countries outside of the U.S.;
- trade protection measures and import or export licensing requirements;
- difficulty in staffing and managing international operations;
- differing labor regulations and business practices;
- potentially negative consequences from changes in or interpretations of tax laws;
- changes in international medical reimbursement policies and programs;
- financial risks such as longer payment cycles, difficulty collecting accounts receivable and exposure to fluctuations in foreign currency exchange rates; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors' and service providers' activities that may fall within the purview of the Foreign Corrupt Practices Act (the FCPA).

Any of these factors may, individually or as a group, have a material adverse effect on our business and results of operations.

As we continue to expand our existing international operations, we may encounter new risks. For example, as we focus on building our international sales and distribution networks in new geographic regions, we must continue to develop relationships with qualified local distributors and trading companies. If we are not successful in developing and maintaining these relationships, we may not be able to grow sales in these geographic regions. These or other similar risks could adversely affect our revenue and profitability.

Our international operations pose currency risks, which may adversely affect our operating results and net income.

A significant and growing portion of our revenues and earnings, as well as our substantial international net assets, are exposed to changes in foreign exchange rates. As we operate in multiple foreign currencies, including the euro, the Brazilian real, the U.K. pound, the Canadian dollar, the Swiss Franc, the Japanese yen and several other currencies, changes in those currencies relative to the U.S. dollar will impact our revenues and expenses. If the U.S. dollar were to weaken against another currency, assuming all other variables remained constant, our revenues would increase, having a positive impact on earnings, and our overall expenses would increase, having a negative impact on earnings. Conversely, if the U.S. dollar were to strengthen against another currency, assuming all other variables remained constant, our revenues would decrease, having a negative impact on earnings, and our overall expenses would decrease, having a positive impact on earnings. In addition, because our financial statements are reported in U.S. dollars, changes in currency exchange rates between the U.S. dollar and other currencies have had, and will continue to have, an impact on our results of operations. Therefore, significant changes in foreign exchange rates can impact our results and our financial guidance.

From time to time, we may implement currency hedges intended to reduce our exposure to changes in foreign currency exchange rates. However, our hedging strategies may not be successful, and any of our unhedged foreign



exchange exposures will continue to be subject to market fluctuations. These risks could cause a material adverse effect on our business, financial position and results of operations and could cause the market value of our common stock to decline.

If we are unable to protect our proprietary technology, we may not be able to compete as effectively.

Where appropriate, we seek patent protection for certain aspects of our technology. Patent protection may not be available for some of the products we are developing. If we must spend significant time and money protecting or enforcing our patents, designing around patents held by others or licensing, potentially for large fees, patents or other proprietary rights held by others, our business and financial prospects may be harmed.

The patent positions of biopharmaceutical products are complex and uncertain. The scope and extent of patent protection for some of our products and product candidates are particularly uncertain because key information on some of our product candidates has existed in the public domain for many years. The composition and genetic sequences of animal and/or human versions of Naglazyme, Aldurazyme and many of our product candidates have been published and are believed to be in the public domain. The chemical structure of 6R-BH4 (the active ingredient in Kuvan) and 3,4-DAP (the active ingredient in Firdapse) have also been published. Publication of this information may prevent us from obtaining or enforcing patents relating to our products and product candidates, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

We own or have licensed patents and patent applications related to Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse. However, these patents and patent applications do not ensure the protection of our intellectual property for a number of reasons, including without limitation the following:

- With respect to pending patent applications, unless and until actually issued, the protective value of these applications is impossible to determine. We do not know whether our patent applications will result in issued patents.
- Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us or that they filed their application for a patent on a claimed invention before we did. Competitors may also claim that we are infringing on their patents and therefore we cannot practice our technology. Competitors may also contest our patents by showing the patent examiner or a court that the invention was not original, was not novel or was obvious, for example. In litigation, a competitor could claim that our issued patents are not valid or are unenforceable for a number of reasons. If a court agrees, we would not be able to enforce that patent. We have no meaningful experience with competitors interfering with or challenging the validity or enforceability of our patents or patent applications.
- Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing products, which could increase our operating expenses and delay product programs. We may not have the financial ability to sustain a patent infringement action, or it may not be financially reasonable to do so.
- Receipt of a patent may not provide much, if any, practical protection. For example, if we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent
- The Leahy-Smith America Invents Act of 2011, which reformed certain patent laws in the U.S., may create additional uncertainty. Among the significant changes are switching from a “first-to-invent” system to a “first-to-file” system, and the implementation of new procedures that permit competitors to challenge our patents in the U.S. Patent and Trademark Office after grant.

It is also unclear whether our trade secrets are adequately protected. Our employees, consultants or contractors may unintentionally or willfully disclose trade secrets to competitors. Enforcing a claim that someone else illegally obtained and is using our trade secrets, as with patent litigation, is expensive and time consuming, requires significant resources and has an unpredictable outcome. In addition, courts outside of the U.S. are sometimes less willing to protect trade secrets. Furthermore, our competitors may independently develop equivalent knowledge, methods and know-how, in which case we would not be able to enforce our trade secret rights against such competitors.

If we are unable to protect our intellectual property, third parties could develop competing products, which could adversely affect our revenue and financial results generally.

\*Competitors and other third parties may have developed intellectual property that could limit our ability to market and commercialize our products and product candidates, if approved.

Similar to us, competitors continually seek intellectual property protection for their technology. Several of our development programs, such as reveglucosidase alfa, vosoritide (formerly referred to as BMN 111) and BMN 270,

focus on therapeutic areas that have been the subject of extensive research and development by third parties for many years. Due to the amount of intellectual property in our field of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or that we will not infringe intellectual property rights of competitors granted or created in the future. For example, if a patent holder believes our product infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe its intellectual property, we would face a number of issues, including the following:

- Defending a lawsuit takes significant executive resources and can be very expensive.
- If a court decides that our product infringes a competitor's intellectual property, we may have to pay substantial damages.

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- With respect to patents, in addition to requiring us to pay substantial damages, a court may prohibit us from making, selling, offering to sell, importing or using our product unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, it may not be available on commercially reasonable terms. For example, we may have to pay substantial royalties or grant cross licenses to our patents and patent applications.
- We may need to redesign our product so it does not infringe the intellectual property rights of others.
- Redesigning our product so it does not infringe the intellectual property rights of competitors may not be possible or could require substantial funds and time.

We may also support and collaborate in research conducted by government organizations, hospitals, universities or other educational institutions. These research partners may be unwilling to grant us any exclusive rights to technology or products derived from these collaborations.

If we do not obtain required licenses or rights, we could encounter delays in our product development efforts while we attempt to design around other patents or may be prohibited from making, using, importing, offering to sell or selling products requiring these licenses or rights. There is also a risk that disputes may arise as to the rights to technology or products developed in collaboration with other parties. If we are not able to resolve such disputes and obtain the licenses or rights we need, we may not be able to develop or market our products.

If our Manufacturing, Marketing and Sales Agreement with Genzyme were terminated, we could be prevented from continuing to commercialize Aldurazyme or our ability to successfully commercialize Aldurazyme would be delayed or diminished.

Either party may terminate the Manufacturing, Marketing and Sales Agreement (the MMS Agreement) between Genzyme and us related to Aldurazyme for specified reasons, including if the other party is in material breach of the MMS Agreement, has experienced a change of control, as such term is defined in the MMS Agreement, or has declared bankruptcy and also is in breach of the MMS Agreement. Although we are not currently in breach of the MMS Agreement, there is a risk that either party could breach the MMS Agreement in the future. Either party may also terminate the MMS Agreement upon one year prior written notice for any reason.

If the MMS Agreement is terminated for breach, the breaching party will transfer its interest in the LLC to the non-breaching party, and the non-breaching party will pay a specified buyout amount for the breaching party's interest in Aldurazyme and in the LLC. If we are the breaching party, we would lose our rights to Aldurazyme and the related intellectual property and regulatory approvals. If the MMS Agreement is terminated without cause, the non-terminating party would have the option, exercisable for one year, to buy out the terminating party's interest in Aldurazyme and in the LLC at a specified buyout amount. If such option is not exercised, all rights to Aldurazyme will be sold and the LLC will be dissolved. In the event of termination of the buyout option without exercise by the non-terminating party as described above, all right and title to Aldurazyme is to be sold to the highest bidder, with the proceeds to be split between Genzyme and us in accordance with our percentage interest in the LLC.

If the MMS Agreement is terminated by either party because the other party declared bankruptcy, the terminating party would be obligated to buy out the other party and would obtain all rights to Aldurazyme exclusively. If the MMS Agreement is terminated by a party because the other party experienced a change of control, the terminating party shall notify the other party, the offeree, of its intent to buy out the offeree's interest in Aldurazyme and the LLC for a stated amount set by the terminating party at its discretion. The offeree must then either accept this offer or agree to buy the terminating party's interest in Aldurazyme and the LLC on those same terms. The party who buys out the other party would then have exclusive worldwide rights to Aldurazyme. The Amended and Restated Collaboration Agreement between us and Genzyme will automatically terminate upon the effective date of the termination of the MMS Agreement and may not be terminated independently from the MMS Agreement.

If we were obligated or given the option to buy out Genzyme's interest in Aldurazyme and the LLC, and thereby gain exclusive rights to Aldurazyme, we may not have sufficient funds to do so and we may not be able to obtain the financing to do so. If we fail to buy out Genzyme's interest, we may be held in breach of the agreement and may lose any claim to the rights to Aldurazyme and the related intellectual property and regulatory approvals. We would then effectively be prohibited from developing and commercializing Aldurazyme. If this happened, not only would our product revenues decrease, but our share price would also decline.

\*If we fail to compete successfully with respect to acquisitions, joint ventures or other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

Our competitors compete with us to attract organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. To date, several of our former and current product programs have been acquired through acquisitions, such as reveglucosidase alfa and talazoparib (sold to Medivation in October 2015) and several of our former and current product programs have been developed through licensing or collaborative arrangements, such as Naglazyme, Aldurazyme, Kuvan and Firdapse. These collaborations include licensing proprietary technology from, and other relationships with, academic research institutions. Our future success will depend, in part, on our ability to identify additional opportunities and to successfully enter into partnering or acquisition agreements for those opportunities. If our competitors successfully enter into partnering arrangements or license agreements with academic research institutions, we will then be precluded from pursuing those specific opportunities. Since each of these opportunities is unique, we may not be able to find a substitute. Several pharmaceutical and biotechnology companies have already established themselves in the field of genetic diseases. These companies have already begun many drug development programs, some of which may target diseases that we are also targeting, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities.

Universities and public and private research institutions also compete with us. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our product candidates. We will attempt to license this proprietary technology, if available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

\*If generic manufacturers use litigation and regulatory means to obtain approval for generic versions of Kuvan, our revenue and results of operations would be adversely affected.

The Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act, permits the FDA to approve ANDAs for generic versions of branded drugs. We refer to this process as the ANDA process. The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient as a branded drug, but does not generally require the conduct and submission of clinical efficacy studies for the generic product. In place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product is bioequivalent to the branded product. Pursuant to the Hatch-Waxman Act, companies were permitted to file ANDA applications for proposed generic versions of Kuvan (sapropterin hydrochloride) at any time after December 2011.

We own several patents that cover Kuvan (sapropterin dihydrochloride), and we have listed those patents in conjunction with that product in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of our Orange Book-listed patents to certify that the applicant believes that our patents are invalid or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify us of such certification (a paragraph IV notice). Upon receipt of a paragraph IV notice, the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the generic's application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA's review of the application may be completed. The discovery, trial and appeals process in such a lawsuit is costly, time consuming,

and may result in generic competition if the ANDA applicant prevails. We have also received three-year Hatch-Waxman exclusivity for a New Patient Population for Kuvan (sapropterin dihydrochloride) that expires in October 2017, including pediatric exclusivity. Thus, depending on the proposed labeling of a generic product, generic versions of Kuvan (sapropterin dihydrochloride) may be prohibited until October 2017, though it is possible that an ANDA applicant could propose to carve out information in the Kuvan labeling protected by the New Patient Population exclusivity and obtain approval earlier.

As previously disclosed, we received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the Orange Book. Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the Orange Book. Together with Merck & Cie, we filed lawsuits against both DRL and Par in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan triggering the automatic 30-month stay on the approval of each ANDA. In response, DRL and Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

As previously disclosed, on September 14, 2015, we entered into a settlement agreement (the Settlement Agreement) with DRL that resolved patent litigation with DRL in the United States described in the above paragraph related to Kuvan (sapropterin dihydrochloride) 100 mg oral tablets. Under the terms of the Settlement Agreement, we will grant DRL a non-exclusive license to its patents related to Kuvan to allow DRL to market a generic version of sapropterin dihydrochloride 100mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from September 18, 2015, or earlier under certain circumstances.

The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending. The Court has not yet set a date for a claim construction hearing or trial in the litigation against Par.

The filing of DRL's and Par's purported ANDAs in respect to Kuvan (sapropterin dihydrochloride) could have an adverse impact on our stock price, and litigation to enforce our patents is likely to cost a substantial amount and require significant management attention. If the patents covering Kuvan (sapropterin dihydrochloride) and its use are not upheld in litigation, or if Par is found to not infringe our asserted patents, the resulting generic competition following the expiration of regulatory exclusivity would have a material adverse effect on our revenue and results of operations. Moreover, generic competition from DRL following the settlement described above could have a material adverse effect on our revenue and results of operations.

If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. From time to time, we publicly announce the expected timing of some of these milestones. All of these milestones are based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. If we do not meet these milestones as publicly announced, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

We depend upon our key personnel and our ability to attract and retain employees.

Our future growth and success will depend in large part on our continued ability to attract, retain, manage and motivate our employees. The loss of the services of any member of our senior management or the inability to hire or retain experienced management personnel could adversely affect our ability to execute our business plan and harm our operating results.



Because of the specialized scientific and managerial nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. In particular, the loss of one or more of our senior executive officers could be detrimental to us if we do not have an adequate succession plan or if we cannot recruit suitable replacements in a timely manner. While our senior executive officers are parties to employment agreements with us, these agreements do not guarantee that they will remain employed with us in the future. In addition, in many cases, these agreements do not restrict our senior executive officers' ability to compete with us after their employment is terminated. The competition for qualified personnel in the pharmaceutical field is intense, and there is a limited pool of qualified potential employees to recruit. Due to this intense competition, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel. If we are unsuccessful in our recruitment and retention efforts, our business may be harmed.

Our success depends on our ability to manage our growth.

Product candidates that we are currently developing or may acquire in the future may be intended for patient populations that are significantly larger than any of Mucopolysaccharidosis I (MPS I), Mucopolysaccharidosis VI (MPS VI), PKU or Lambert Eaton Myasthenic Syndrome (LEMS). In order to continue development and marketing of these products, if approved, we will need to significantly expand our operations. To manage expansion effectively, we need to continue to develop and improve our research and development capabilities, manufacturing and quality capacities, sales and marketing capabilities, financial and administrative systems and standard processes for global operations. Our staff, financial resources, systems, procedures or controls may be inadequate to support our operations and may increase our exposure to regulatory and corruption risks and our management may be unable to manage successfully future market opportunities or our relationships with customers and other third-parties.

Changes in methods of treatment of disease could reduce demand for our products and adversely affect revenues.

Even if our drug products are approved, if doctors elect a course of treatment which does not include our drug products, this decision would reduce demand for our drug products and adversely affect revenues. For example, if gene therapy becomes widely used as a treatment of genetic diseases, the use of enzyme replacement therapy, such as Naglazyme, Vimizim, and Aldurazyme in MPS diseases, could be greatly reduced. Changes in treatment method can be caused by the introduction of other companies' products or the development of new technologies or surgical procedures which may not directly compete with ours, but which have the effect of changing how doctors decide to treat a disease.

\*If product liability lawsuits are successfully brought against us, we may incur substantial liabilities.

We are exposed to the potential product liability risks inherent in the testing, manufacturing and marketing of human pharmaceuticals. We currently maintain insurance against product liability lawsuits for the commercial sale of our products and for the clinical trials of our product candidates. Pharmaceutical companies must balance the cost of insurance with the level of coverage based on estimates of potential liability. Historically, the potential liability associated with product liability lawsuits for pharmaceutical products has been unpredictable. Although we believe that our current insurance is a reasonable estimate of our potential liability and represents a commercially reasonable balancing of the level of coverage as compared to the cost of the insurance, we may be subject to claims in connection with our clinical trials and commercial use of our products and product candidates for which our insurance coverage may not be adequate and we may be unable to avoid significant liability if any product liability lawsuit is brought against us. If we are the subject of a successful product liability claim that exceeds the limits of any insurance coverage we obtain, we may incur substantial charges that would adversely affect our earnings and require the commitment of capital resources that might otherwise be available for the development and commercialization of our product programs.

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.

We rely significantly on our information technology and manufacturing infrastructure to effectively manage and maintain our inventory and internal reports, to manufacture and ship products to customers and to timely invoice them. Any failure, inadequacy or interruption of that infrastructure or security lapse of that technology, including cybersecurity incidents could harm our ability to operate our business effectively. Our ability to manage and maintain our inventory and internal reports, to manufacture and ship our products to customers and timely invoice them depends significantly on our enterprise resource planning, production management and other information systems. Cybersecurity attacks in particular are evolving and include, but are not limited to, malicious software, attempts to gain unauthorized access to data and other electronic security breaches that could lead to disruptions in systems,

misappropriation of our confidential or otherwise protected information and corruption of data. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access of these systems, may affect our ability to manage and maintain our inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary and confidential information, including research or clinical data, could require significant capital investments to remediate and could adversely affect our business, financial condition and results of operations.

\*Our business is affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates and foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to pass through price increases on to our customers due to the process by which health care providers are reimbursed for our products by the government. Interest rates, the liquidity of the credit markets and the volatility of the capital markets could also affect the value of our investments and our ability to liquidate our investments in order to fund our operations. We purchase or enter into a variety of financial instruments and transactions, including investments in commercial paper, the extension of credit to corporations, institutions and governments and hedging contracts. If any of the issuers or counter parties to these instruments were to default on their obligations, it could materially reduce the value of the transaction and adversely affect our cash flows.

For each of the three and nine months ended September 30, 2015, 4% of our net product revenues were from Italy, Spain, Portugal, Greece and Russia. Approximately 8% of our total accounts receivable as of September 30, 2015 related to such countries and we have included an allowance for doubtful accounts for certain accounts receivable from Greece. If the financial conditions of these countries continues to decline, a substantial portion of the receivables may be uncollectable, which would mean we would have to provide for additional allowances for doubtful accounts or cease selling products in these countries, either of which could adversely affect our results of operations. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

Interest rates and the ability to access credit markets could also adversely affect the ability of our customers/distributors to purchase, pay for and effectively distribute our products. Similarly, these macroeconomic factors could affect the ability of our contract manufacturers, sole-source or single-source suppliers to remain in business or otherwise manufacture or supply product. Failure by any of them to remain a going concern could affect our ability to manufacture products.

Recent and future regulatory actions and other events may adversely affect the trading price and liquidity of our senior subordinated convertible notes.

We expect that many investors in, and potential purchasers of, the Notes will employ, or seek to employ, a convertible arbitrage strategy with respect to the Notes. Investors would typically implement such a strategy by selling short the common stock underlying the Notes and dynamically adjusting their short position while continuing to hold the Notes. Investors may also implement this type of strategy by entering into swaps on our common stock in lieu of or in addition to short selling the common stock.

The SEC and other regulatory and self-regulatory authorities have implemented various rules and taken certain actions, and may in the future adopt additional rules and take other actions, that may impact those engaging in short selling activity involving equity securities (including our common stock). Such rules and actions include Rule 201 of SEC Regulation SHO, the adoption by the Financial Industry Regulatory Authority, Inc. of a "Limit Up-Limit Down" program, the imposition of market-wide circuit breakers that halt trading of securities for certain periods following specific market declines, and the implementation of certain regulatory reforms required by the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010. Any governmental or regulatory action that restricts the ability of investors in, or potential purchasers of, the Notes to effect short sales of our common stock or enter into swaps on

our common stock could adversely affect the trading price and the liquidity of the Notes.

In addition, if investors and potential purchasers seeking to employ a convertible arbitrage strategy are unable to borrow or enter into swaps on our common stock, in each case on commercially reasonable terms, the trading price and liquidity of the Notes may be adversely affected.

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\*Risks Related to our Pending Acquisition of Rights to Kuvan and Pegvaliase (the Phenylketonuria (PKU) Franchise) from Merck Serono

\* If the pending transaction with Merck Serono is completed and we are unable to successfully integrate the expanded PKU franchise into our existing business operations, our business could be adversely affected.

We will need to successfully integrate the PKU franchise rights we expect to acquire from Merck Serono with our other business operations. Previously, we had exclusive rights to Kuvan in the United States and Canada and to pegvaliase in the United States and Japan. After the closing of the transaction with Merck Serono, we will have exclusive worldwide rights to Kuvan and pegvaliase, with the exception of Kuvan in Japan. Integrating the expanded PKU business with our existing business will be a complex and time-consuming process. There may be substantial difficulties, costs and delays involved in any integration of the expanded PKU business with that of our existing operations. These may include:

- distracting management from day-to-day operations;
- an inability to achieve synergies as planned;
- changes in the acquired rights due to potential divestitures or other requirements imposed by antitrust regulators;
- costs and delays in transitioning activities from Merck Serono, particularly with respect to the transfer of the Kuvan marketing authorizations;
- impact of unforeseen country level regulatory changes, delays and actions;
- difficulties in establishing distribution arrangements for Kuvan in all territories;
- reliance on Merck Serono to provide critical transition services for sales and distribution of Kuvan until marketing authorizations can be transferred in all countries;
- difficulties with respect to the timing and results of ongoing and future clinical trials of pegvaliase;
- an inability to manufacture both Kuvan and pegvaliase (pending regulatory approval) in the quantity and configuration required for each jurisdiction and intended use; and
- increased challenges in managing our business due to the global expansion of the PKU franchise.

Many of these risks may be accentuated because the acquired rights relate to activities outside of the U.S. Any one or all of these factors may increase operating costs or lower anticipated financial performance. Many of these factors are also outside of our control. Achieving anticipated synergies and the potential benefits underlying our reasons for the acquisition will depend on successful integration of the PKU franchise rights. The failure to integrate the expanded PKU business successfully would have a material adverse effect on our business, financial condition and results of operations.

\*The actual impact of the acquisition of the PKU franchise rights on our financial results may be worse than the assumptions we have used.

Even if the integration of the PKU franchise rights is successful, we have made certain assumptions relating to the impact on our financial results in respect of the acquisition. These assumptions relate to numerous matters, including:

- the amount of goodwill and intangible assets that will result from the acquisition;
- acquisition costs, including transaction and integration costs; and
- other financial and strategic risks of the acquisition.

Irrespective of our assumptions, we may incur higher than expected operating, transaction and integration costs, and we may encounter general economic and business conditions that adversely affect us following the acquisition. If one or more of these assumptions are incorrect, it could have an adverse effect on our business and operating results, and the perceived benefits from the acquisition may not be realized.



\*We will incur significant transaction and integration costs in connection with the acquisition of the PKU franchise rights.

We have incurred significant transaction costs related to the acquisition of the PKU franchise rights, and will continue to incur such costs through the closing of the transaction. In addition, the combined business will incur integration costs following the completion of the acquisition as we integrate the expanded PKU business with our other businesses. Although we expect that the realization of benefits and efficiencies related to the integration of the businesses may offset over time these transaction and integration costs, no assurances can be made that this net benefit will be achieved in the near term, or at all, which could adversely affect our financial condition and results of operations.

#### Risks Related to our Acquisition of Prosensa Holding N.V.

If we do not successfully integrate Prosensa into our business operations, our business could be adversely affected.

We will need to successfully integrate the operations of Prosensa with our business operations. Integrating the operations of Prosensa with that of our own will be a complex and time-consuming process. Prior to the acquisition, Prosensa operated independently, with its own business, corporate culture, locations, employees and systems. There may be substantial difficulties, costs and delays involved in any integration of the business of Prosensa with that of our own. These may include:

- distracting management from day-to-day operations;
- potential incompatibility of corporate cultures;
- an inability to achieve synergies as planned;
- changes in the combined business due to potential divestitures or other requirements imposed by antitrust regulators;
- costs and delays in implementing common systems and procedures; and
- increased difficulties in managing our business due to the addition of international locations.

Many of these risks may be accentuated because the majority of Prosensa's operations, employees and customers are located outside of the U.S. Any one or all of these factors may increase operating costs or lower anticipated financial performance. Many of these factors are also outside of our control. Achieving anticipated synergies and the potential benefits underlying our reasons for the acquisition will depend on successful integration of the businesses. The failure to integrate the business operations of Prosensa successfully would have a material adverse effect on our business, financial condition and results of operations.

The actual impact of the acquisition of Prosensa on our capital structure and financial results may be worse than the assumptions we have used.

Even if the integration is successful, we have made certain assumptions relating to the impact on our capital structure and financial results in respect of the acquisition. These assumptions relate to numerous matters, including:

- our expected capital structure after the acquisition;
- the amount of goodwill and intangibles that will result from the acquisition;
- certain other purchase accounting adjustments that we expect will be recorded in our financial statements in connection with the acquisition;
- acquisition costs, including restructuring charges and transaction costs; and
- other financial and strategic risks of the acquisition.

Irrespective of our assumptions, we may incur higher than expected operating, transaction and integration costs, and we may encounter general economic and business conditions that adversely affect the combined company following



the acquisition. If one or more of these assumptions are incorrect, it could have an adverse effect on our business and operating results, and the perceived benefits from the acquisition may not be realized.

We may have exposure to additional tax liabilities as a result of the acquisition of Prosensa.

As a multinational corporation, we are subject to income taxes as well as non-income based taxes, in both the U.S. and various foreign jurisdictions. Significant judgment is required in determining our worldwide provision for income taxes and other tax liabilities. Changes in tax laws or tax rulings may have a significantly adverse impact on our effective tax rate. Proposals by the current U.S. administration for fundamental U.S. international tax reform, including without limitation provisions that would limit the ability of U.S. multinationals to defer U.S. taxes on foreign income, if enacted, could have a significant adverse impact on our effective tax rate following the acquisition.

We are subject to a variety of additional risks as a result of the acquisition of Prosensa that may negatively impact our operations.

As a result of the acquisition, we are subject to new and additional risks associated with the business and operations of Prosensa and its global operations. The additional risks we may be exposed to include but are not limited to the following:

- tariffs and trade barriers;
- regulations related to customs and import/export matters (including sanctions);
- longer payment cycles;
- tax issues, such as tax law changes and variations in tax laws as compared to the jurisdictions in which we already operate;
- operating under regulations in new jurisdictions related to obtaining eligibility for government or private payer reimbursement for our products at the wholesale/retail level;
  - cultural and language differences in the new jurisdictions in which we will operate;
- complying with additional employment regulations in the new jurisdictions in which we will operate; and
- risks related to crimes, strikes, riots, civil disturbances, terrorist attacks and wars in new geographical locations.

We cannot assure you that we will be able to adequately address these additional risks. If we are unable to do so, our operations might suffer.

Additionally, although prior to the acquisition we had international operations, as a result of the acquisition, we operate on an expanded global basis with additional offices or activities in Europe. We will face increased exposure to risks inherent in conducting business internationally, including compliance with international laws and regulations and laws and regulations of the U.S. and various other countries that apply to our international operations. Compliance with these laws and regulations may increase our cost of doing business in foreign jurisdictions. These laws and regulations include laws relating to the pharmaceutical industry, data privacy requirements, labor relations laws, tax laws, anti-competition regulations, import and trade restrictions, export requirements, U.S. laws such as the FCPA, other U.S. federal statutes and regulations, including those established by the Office of Foreign Assets Control, and local laws which prohibit payments to governmental officials. Given the high level of complexity of these laws, however, there is a risk that some provisions may be inadvertently breached by us, for example through fraudulent or negligent behavior of individual employees, our failure to comply with certain formal documentation requirements, or otherwise. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers or our employees, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, our business and our operating results. Our success depends, in part, on our ability to anticipate these risks and manage these challenges. These factors or any combination of these factors may adversely affect our revenue or our overall financial performance.

We will incur significant transaction, integration and restructuring costs in connection with the acquisition of Prosensa.

We have incurred significant transaction costs related to the acquisition. In addition, the combined business will incur integration and restructuring costs following the completion of the acquisition as we integrate Prosensa's businesses with our businesses. Although we expect that the realization of benefits and efficiencies related to the integration of the businesses may offset over time these transaction and integration and restructuring costs, no assurances can be made that this net benefit will be achieved in the near term, or at all, which could adversely affect our financial condition and results of operations.

Prosensa depends heavily on the success of Kyndrisa, formerly referred to as drisapersen. Kyndrisa is still in clinical development. If we are unable to commercialize Kyndrisa or experience significant delays in doing so, our business, financial condition and results of operations will be materially adversely affected.

Our ability to generate product revenues from Prosensa will depend heavily on the successful development and eventual commercialization of Kyndrisa.

In September 2013, Prosensa announced that the Phase 3 clinical trial of Kyndrisa did not meet its primary endpoint. Although we believe that the collective data from Prosensa's various Phase 2 and Phase 3 clinical trials of Kyndrisa, including retrospective and subgroup analyses, provide strong support for concluding that Kyndrisa showed clinically meaningful improvements over placebo in these trials, we cannot be sure that Prosensa's data will be sufficient to satisfy the EMA or the FDA. We may need to conduct additional clinical trials at significant delay and cost or abandon development of Kyndrisa altogether.

Even if we receive regulatory approval for and are able to commercialize Kyndrisa, our success will be subject to the following risks:

- we may not achieve market acceptance of Kyndrisa by physicians, patients and third-party payers;
- Kyndrisa may not have an acceptable safety profile following approval;
- we may not be able to manufacture Kyndrisa in compliance with requirements of the EMA, the FDA and similar regulatory agencies in commercial quantities sufficient to meet market demand;
- we may not achieve sufficient pricing for Kyndrisa to compensate for future development and commercialization costs and to recoup our cost to acquire Prosensa;
- we may not compete successfully with any alternative therapies for Duchenne muscular dystrophy (DMD); and
- we may not successfully enforce and defend our intellectual property rights and claims.

The occurrence of any of these events could materially adversely affect our business, financial condition and results of operations.

Our conclusions regarding the efficacy of Kyndrisa are based on retrospective analyses of the results of Prosensa's clinical trials, and these analyses may be considered less reliable indicators of efficacy than pre-specified analyses.

After determining that it did not achieve the primary efficacy endpoint in the completed Phase 3 clinical trial of Kyndrisa, Prosensa performed retrospective and subgroup analyses of the Phase 3 clinical trial and prior Phase 2 clinical trials of Kyndrisa that we believe provide strong support for concluding that Kyndrisa showed clinically meaningful improvements over placebo in these trials. Although Prosensa believed that these additional analyses were warranted, a retrospective analysis performed after unblinding trial results can result in the introduction of bias if the analysis is inappropriately tailored or influenced by knowledge of the data and actual results. Because of these limitations, regulatory authorities typically give greatest weight to results from pre-specified analyses and less weight to results from post-hoc, retrospective analyses. Thus, this increases the likelihood that we will have to conduct an additional clinical trial or trials of Kyndrisa before we can apply for marketing approval.

Because Prosensa was developing product candidates for the treatment of diseases in which there is little clinical experience and, in some cases, using new endpoints or methodologies, there is more risk that the outcome of clinical trials for Prosensa's product candidates will not be favorable.

There is currently no approved disease-modifying therapy for DMD. In addition, there has been limited historical clinical trial experience generally for the development of drugs to treat the underlying cause of DMD. As a result, the design and conduct of clinical trials for this disease, particularly for drugs to address the underlying cause of this disease, are subject to increased risks. In particular, regulatory authorities in the U.S. and the EU have not issued

definitive guidance as to how to measure and achieve efficacy.

In the last several years, the six-minute walk test (6MWT) has been used in several trials of product candidates for patients with DMD, and is accepted by U.S. and European regulators to be an appropriate primary outcome measure for DMD trials. Because of the limited clinical experience in this indication however, regulators have not yet established what difference in the six-minute walk distance (6MWD) is required to be demonstrated in a clinical trial of a DMD therapy in order to signify a clinically meaningful result and/or obtain regulatory approvals. As a result, it is not clear what is required in terms of 6MWD or other end points to obtain regulatory approval for Kyndrisa and our other product candidates acquired from Prosensa. If we are required to conduct additional clinical trials of Kyndrisa, the design of such trials could be subject to such uncertainties.

We could also face similar challenges in designing clinical trials and obtaining regulatory approval for future product candidates, including any that we may develop for myotonic dystrophy or Huntington's disease because there is also limited historical clinical trial experience for the development of drugs to treat these diseases.

#### Risks Related to Ownership of Our Securities

\*Our stock price may be volatile, and an investment in our stock could suffer a decline in value.

Our valuation and stock price since the beginning of trading after our initial public offering have had no meaningful relationship to current or historical earnings, asset values, book value or many other criteria based on conventional measures of stock value. The market price of our common stock will fluctuate due to factors including:

- product sales and profitability of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- manufacturing, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- progress of our integration of Prosensa and the PKU franchise rights to be acquired from Merck Serono;
- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;
- results relating to our lawsuit against Par to protect our patents relating to Kuvan and generic competition to Kuvan relating to our settlement with DRL;
- government regulatory action affecting our product candidates or our competitors' drug products in both the U.S. and non-U.S. countries;
- developments or disputes concerning patent or proprietary rights;
- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;
- economic conditions in the U.S. or abroad;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results; and
- changes in company assessments or financial estimates by securities analysts.

In the past, following periods of large price declines in the public market price of a company's securities, securities class action litigation has often been initiated against that company. Litigation of this type could result in substantial costs and diversion of management's attention and resources, which would hurt our business. Any adverse determination in litigation could also subject us to significant liabilities. In addition, our stock price can be materially adversely affected by factors beyond our control, such as disruptions in global financial markets or negative trends in the biotechnology sector of the economy, even if our business is operating well.

Anti-takeover provisions in our charter documents and under Delaware law may make an acquisition of us, which may be beneficial to our stockholders, more difficult.

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law and our charter documents as currently in effect may make a change in control of our company more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our certificate of incorporation providing that stockholders' meetings may only be called by our Board of Directors and provisions in our bylaws providing that the stockholders may not take action by written consent and requiring that stockholders that desire to nominate any person for election to our Board of Directors or to make any proposal with respect to business to be conducted at a meeting of our stockholders be submitted in appropriate form to our Secretary within a specified period of time in advance of any such meeting. Additionally, our Board of Directors has the authority to issue shares of preferred stock and to determine the terms of those shares of stock without any further action by our stockholders. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, our Board of Directors approves the transaction. Our Board of Directors may use these provisions to prevent changes in the management and control of our company. Also, under applicable Delaware law, our Board of Directors may adopt additional anti-takeover measures in the future.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

None

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures

None.

Item 5. Other Information.

None.





Item 6. Exhibits.

- 10.1 Asset Purchase Agreement between BioMarin Pharmaceutical Inc. and Medivation, Inc., dated August 21, 2015, previously filed with the SEC on October 7, 2015 as Exhibit 2.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.
- 10.2\* Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck & Cie, Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd., dated September 14, 2015. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.
- 31.1\* Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
- 31.2\* Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
- 32.1\* Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.
- 101.INS\* XBRL Instance Document
- 101.SCH\* XBRL Taxonomy Extension Schema Document
- 101.CAL\* XBRL Taxonomy Extension Calculation Document
- 101.DEF\* XBRL Taxonomy Extension Definition Linkbase
- 101.LAB\* XBRL Taxonomy Extension Labels Linkbase Document
- 101.PRE\* XBRL Taxonomy Extension Presentation Link Document

\*Filed herewith.

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets as of September 30, 2015 and December 31, 2014, (ii) Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2015 and 2014, (iii) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2015 and 2014, and (iv) Notes to Condensed Consolidated Financial Statements.



SIGNATURE

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.

BIOMARIN PHARMACEUTICAL INC.

Dated: November 2, 2015 By /S/ DANIEL SPIEGELMAN

Daniel Spiegelman,

Executive Vice President and Chief Financial Officer  
(On behalf of the registrant and as principal financial officer)

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