

BIOMARIN PHARMACEUTICAL INC

Form 10-Q

October 28, 2014

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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, 2014**

**Or**

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

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

**Commission File Number: 000-26727**

**BioMarin Pharmaceutical Inc.**

**(Exact name of registrant as specified in its charter)**

**Delaware**  
**(State or other jurisdiction of**  
**incorporation or organization)**

**68-0397820**  
**(I.R.S. Employer**  
**Identification No.)**

**770 Lindero Street, San Rafael, California**  
**(Address of principal executive offices)**  
**(415) 506-6700**

**94901**  
**(Zip Code)**

**(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: 147,465,546 shares of common stock, par value \$0.001, outstanding as of October 18, 2014.



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**BIOMARIN PHARMACEUTICAL INC.**

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BioMarin®, Naglazyme®, Kuvan® and VIMIZIM® are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC. Firdapse is a trademark of BioMarin Pharmaceutical Inc., or its affiliates.

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**BIOMARIN PHARMACEUTICAL INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**

**September 30, 2014 and December 31, 2013**

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

|                                                                                           | <b>September 30,<br/>2014</b> | <b>December 31,<br/>2013 <sup>(1)</sup></b> |
|-------------------------------------------------------------------------------------------|-------------------------------|---------------------------------------------|
|                                                                                           | (unaudited)                   |                                             |
| <b>ASSETS</b>                                                                             |                               |                                             |
| Current assets:                                                                           |                               |                                             |
| Cash and cash equivalents                                                                 | \$ 398,005                    | \$ 568,781                                  |
| Short-term investments                                                                    | 268,368                       | 215,942                                     |
| Accounts receivable, net (allowance for doubtful accounts: \$195 and \$529, respectively) | 121,395                       | 117,822                                     |
| Inventory                                                                                 | 204,444                       | 162,605                                     |
| Current deferred tax assets                                                               | 29,149                        | 30,561                                      |
| Other current assets                                                                      | 56,458                        | 41,707                                      |
| Total current assets                                                                      | 1,077,819                     | 1,137,418                                   |
| Noncurrent assets:                                                                        |                               |                                             |
| Investment in BioMarin/Genzyme LLC                                                        | 815                           | 816                                         |
| Long-term investments                                                                     | 448,328                       | 267,700                                     |
| Property, plant and equipment, net                                                        | 486,741                       | 319,316                                     |
| Intangible assets, net                                                                    | 159,949                       | 163,147                                     |
| Goodwill                                                                                  | 54,258                        | 54,258                                      |
| Long-term deferred tax assets                                                             | 150,991                       | 145,234                                     |
| Other assets                                                                              | 61,532                        | 156,171                                     |
| Total assets                                                                              | \$ 2,440,433                  | \$ 2,244,060                                |
| <b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>                                               |                               |                                             |
| Current liabilities:                                                                      |                               |                                             |
| Accounts payable and accrued liabilities                                                  | \$ 206,550                    | \$ 183,271                                  |
| Total current liabilities                                                                 | 206,550                       | 183,271                                     |
| Noncurrent liabilities:                                                                   |                               |                                             |
| Long-term convertible debt                                                                | 656,884                       | 655,566                                     |
| Long-term contingent acquisition consideration payable                                    | 42,297                        | 30,790                                      |
| Other long-term liabilities                                                               | 26,260                        | 33,392                                      |
| Total liabilities                                                                         | 931,991                       | 903,019                                     |

## Stockholders' equity:

|                                                                                                                                                                                                                                  |    |           |     |           |
|----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|----|-----------|-----|-----------|
| Common stock, \$0.001 par value: 250,000,000 shares authorized at September 30, 2014 and December 31, 2013: 147,433,951 and 143,463,668 shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively. |    |           | 148 | 144       |
| Additional paid-in capital                                                                                                                                                                                                       |    | 2,278,761 |     | 2,059,101 |
| Company common stock held by Nonqualified Deferred Compensation Plan                                                                                                                                                             |    | (9,683)   |     | (7,421)   |
| Accumulated other comprehensive income                                                                                                                                                                                           |    | 19,189    |     | 5,018     |
| Accumulated deficit                                                                                                                                                                                                              |    | (779,973) |     | (715,801) |
| Total stockholders' equity                                                                                                                                                                                                       |    | 1,508,442 |     | 1,341,041 |
| Total liabilities and stockholders' equity                                                                                                                                                                                       | \$ | 2,440,433 | \$  | 2,244,060 |

- (1) December 31, 2013 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, 2013, filed with the Securities and Exchange Commission (the SEC) on February 26, 2014.

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

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|                                                                             | <b>Three Months Ended<br/>September 30,</b> |                    | <b>Nine Months Ended<br/>September 30,</b> |                     |
|-----------------------------------------------------------------------------|---------------------------------------------|--------------------|--------------------------------------------|---------------------|
|                                                                             | <b>2014</b>                                 | <b>2013</b>        | <b>2014</b>                                | <b>2013</b>         |
| <b>REVENUES:</b>                                                            |                                             |                    |                                            |                     |
| Net product revenues                                                        | \$ 173,416                                  | \$ 134,330         | \$ 510,664                                 | \$ 394,074          |
| Collaborative agreement revenues                                            | 353                                         | 1,754              | 1,274                                      | 2,778               |
| Royalty, license and other revenues                                         | 3,078                                       | 790                | 8,248                                      | 4,760               |
| Total revenues                                                              | 176,847                                     | 136,874            | 520,186                                    | 401,612             |
| <b>OPERATING EXPENSES:</b>                                                  |                                             |                    |                                            |                     |
| Cost of sales (excludes amortization of certain acquired intangible assets) | 29,920                                      | 28,054             | 83,946                                     | 71,121              |
| Research and development                                                    | 125,686                                     | 88,064             | 319,554                                    | 257,468             |
| Selling, general and administrative                                         | 74,604                                      | 61,841             | 202,524                                    | 163,547             |
| Intangible asset amortization and contingent consideration                  | 2,934                                       | 9,639              | 15,797                                     | 13,173              |
| Gain on sale of intangible asset                                            | (67,500)                                    | 0                  | (67,500)                                   | 0                   |
| Total operating expenses                                                    | 165,644                                     | 187,598            | 554,321                                    | 505,309             |
| <b>INCOME (LOSS) FROM OPERATIONS</b>                                        | <b>11,203</b>                               | <b>(50,724)</b>    | <b>(34,135)</b>                            | <b>(103,697)</b>    |
| Equity in the loss of BioMarin/Genzyme LLC                                  | (225)                                       | (147)              | (1,102)                                    | (711)               |
| Interest income                                                             | 1,435                                       | 574                | 4,293                                      | 1,942               |
| Interest expense                                                            | (9,118)                                     | (526)              | (27,445)                                   | (2,854)             |
| Debt conversion expense                                                     | 0                                           | (1,732)            | (674)                                      | (12,152)            |
| Other income (expense)                                                      | (74)                                        | 239                | (68)                                       | 344                 |
| <b>INCOME (LOSS) BEFORE INCOME TAXES</b>                                    | <b>3,221</b>                                | <b>(52,316)</b>    | <b>(59,131)</b>                            | <b>(117,128)</b>    |
| Provision for (benefit from) income taxes                                   | (4,224)                                     | 704                | 5,041                                      | (2,765)             |
| <b>NET INCOME (LOSS)</b>                                                    | <b>\$ 7,445</b>                             | <b>\$ (53,020)</b> | <b>\$ (64,172)</b>                         | <b>\$ (114,363)</b> |
| <b>NET INCOME (LOSS) PER SHARE, BASIC</b>                                   | <b>\$ 0.05</b>                              | <b>\$ (0.38)</b>   | <b>\$ (0.44)</b>                           | <b>\$ (0.84)</b>    |
| <b>NET INCOME (LOSS) PER SHARE, DILUTED</b>                                 | <b>\$ 0.05</b>                              | <b>\$ (0.38)</b>   | <b>\$ (0.44)</b>                           | <b>\$ (0.84)</b>    |

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|                                                     |         |         |         |         |
|-----------------------------------------------------|---------|---------|---------|---------|
| Weighted average common shares outstanding, basic   | 147,016 | 140,796 | 145,724 | 136,102 |
| Weighted average common shares outstanding, diluted | 159,304 | 140,796 | 145,724 | 136,102 |

|                                    |           |             |             |              |
|------------------------------------|-----------|-------------|-------------|--------------|
| <b>COMPREHENSIVE INCOME (LOSS)</b> | \$ 16,693 | \$ (43,988) | \$ (50,001) | \$ (102,688) |
|------------------------------------|-----------|-------------|-------------|--------------|

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



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**BIOMARIN PHARMACEUTICAL INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS**

**Nine Months Ended September 30, 2014 and 2013**

**(In thousands of U.S. dollars)**

**(Unaudited)**

|                                                                                    | <b>2014</b> | <b>2013</b>  |
|------------------------------------------------------------------------------------|-------------|--------------|
| <b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>                                       |             |              |
| Net loss                                                                           | \$ (64,172) | \$ (114,363) |
| Adjustments to reconcile net loss to net cash used in operating activities:        |             |              |
| Depreciation and amortization                                                      | 39,921      | 37,528       |
| Non-cash interest expense                                                          | 20,305      | 352          |
| Accretion of discount on investments                                               | 5,748       | 4,149        |
| Stock-based compensation                                                           | 55,251      | 42,638       |
| Gain on sale of intangible asset                                                   | (67,500)    | 0            |
| Gain on termination of lease                                                       | (8,893)     | 0            |
| Equity in the loss of BioMarin/Genzyme LLC                                         | 1,102       | 711          |
| Deferred income taxes                                                              | (12,373)    | (19,212)     |
| Excess tax benefit from stock option exercises                                     | (205)       | (335)        |
| Unrealized foreign exchange (gain) loss on forward contracts                       | 2,354       | (1,692)      |
| Non-cash changes in the fair value of contingent acquisition consideration payable | 11,202      | 9,816        |
| Debt conversion expense                                                            | 674         | 12,152       |
| Other non-cash movements                                                           | 360         | 939          |
| Changes in operating assets and liabilities:                                       |             |              |
| Accounts receivable, net                                                           | (3,573)     | (15,679)     |
| Inventory                                                                          | (41,839)    | (19,989)     |
| Other current assets                                                               | (9,783)     | (1,776)      |
| Other assets                                                                       | (6,718)     | (307)        |
| Accounts payable and accrued liabilities                                           | 31,493      | 11,362       |
| Other long-term liabilities                                                        | (116)       | 5,368        |
| Net cash used in operating activities                                              | (46,762)    | (48,338)     |
| <b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>                                       |             |              |
| Purchases of property, plant and equipment                                         | (89,628)    | (35,440)     |
| Maturities and sales of investments                                                | 207,476     | 232,625      |
| Purchase of available-for-sale investments                                         | (448,938)   | (179,192)    |
| Proceeds from sale of intangible asset                                             | 67,500      | 0            |
| Business acquisitions, net of cash acquired                                        | 0           | (9,875)      |
| Investment in BioMarin/Genzyme LLC                                                 | (1,100)     | (485)        |
| Other                                                                              | (2,000)     | 0            |

|                                                     |           |       |
|-----------------------------------------------------|-----------|-------|
| Net cash (used in) provided by investing activities | (266,690) | 7,633 |
|-----------------------------------------------------|-----------|-------|

**CASH FLOWS FROM FINANCING ACTIVITIES:**

|                                                             |         |          |
|-------------------------------------------------------------|---------|----------|
| Proceeds from exercises of stock options                    | 37,635  | 60,229   |
| Taxes paid related to net share settlement of equity awards | (7,246) | (6,141)  |
| Proceeds from public offering of common stock, net          | 117,463 | 0        |
| Excess tax benefit from stock option exercises              | 205     | 335      |
| Payments for debt conversion                                | (674)   | (12,152) |
| Payment of contingent acquisition consideration payable     | (4,691) | 0        |
| Other                                                       | (16)    | (528)    |

|                                           |         |        |
|-------------------------------------------|---------|--------|
| Net cash provided by financing activities | 142,676 | 41,743 |
|-------------------------------------------|---------|--------|

**NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS**

|                            |            |            |
|----------------------------|------------|------------|
|                            | (170,776)  | 1,038      |
| Cash and cash equivalents: |            |            |
| Beginning of period        | \$ 568,781 | \$ 180,527 |
| End of period              | \$ 398,005 | \$ 181,565 |

**SUPPLEMENTAL CASH FLOW DISCLOSURES:**

|                                                                       |          |          |
|-----------------------------------------------------------------------|----------|----------|
| Cash paid for interest, net of interest capitalized into fixed assets | \$ 4,759 | \$ 3,238 |
| Cash paid for income taxes                                            | 22,378   | 13,165   |
| Stock-based compensation capitalized into inventory                   | 5,663    | 4,219    |
| Depreciation capitalized into inventory                               | 7,989    | 8,221    |

**SUPPLEMENTAL CASH FLOW DISCLOSURES FROM INVESTING AND FINANCING ACTIVITIES:**

|                                                                                                                    |            |            |
|--------------------------------------------------------------------------------------------------------------------|------------|------------|
| Decrease in accounts payable and accrued liabilities related to fixed assets                                       | \$ (2,762) | \$ (7,491) |
| Conversion of convertible debt                                                                                     | 16,482     | 269,816    |
| Deferred offering costs reclassified into additional paid-in-capital as a result of conversion of convertible debt | 126        | 2,618      |
| Release of escrow balance for purchase of San Rafael Corporate Center                                              | 116,500    | 0          |

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

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**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 investigational product candidates. The Company's approved products are VIMIZIM (elosulfase alpha), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

Through September 30, 2014, the Company had accumulated losses of approximately \$780.0 million. 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, if approved; the uncertainty of the Company's research and development 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. 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, 2013 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, 2014 are

not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2014.

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.

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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)**

**(3) SIGNIFICANT ACCOUNTING POLICIES**

There have been no material changes to the Company's significant accounting policies during the nine months ended September 30, 2014, 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, 2013.

***Reclassifications***

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

**(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, 2014, as compared to the recent accounting pronouncements described in the Company's Annual Report on Form 10-K for the year-ended December 31, 2013, that are of significance or potential significance to the Company.

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update 2014-09 (ASU 2014-09), *Revenue from Contracts with Customers*. 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, 2016, including interim periods within that reporting period, which for the Company is January 1, 2017. Early adoption is not permitted. The Company is currently evaluating the potential impact the adoption of ASU 2014-09 will have on its consolidated financial statements.

**(5) ACQUISITION OF 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 transaction, 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 purchase price for SRCC was \$116.5 million. The fair value of the consideration paid was \$116.5 million, all of which was paid in cash, which was held in escrow as of December 31, 2013.

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

|                                      | <b>Estimated<br/>Fair Value</b> | <b>Estimated Useful Lives</b> |
|--------------------------------------|---------------------------------|-------------------------------|
| Building and improvements            | \$ 94,414                       | 50 years                      |
| Land                                 | 14,565                          |                               |
| Land improvements                    | 3,616                           | 10 years                      |
| Intangible assets                    | 3,905                           | Remaining lease term          |
| <b>Total identifiable net assets</b> | <b>\$ 116,500</b>               |                               |

Table of Contents**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 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 intangible assets acquired by asset class as of the date of acquisition:

|                                                        |                 |
|--------------------------------------------------------|-----------------|
| Above market leases                                    | \$ 351          |
| In-place leases                                        | 3,554           |
| <b>Total intangible assets subject to amortization</b> | <b>\$ 3,905</b> |

The value of any in-place 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 in-place lease intangible assets to expense over the remaining initial term of each lease. The Company will amortize the capitalized value of above market leases to expense over the remaining lives of the underlying leases.

The amount of third-party tenant revenue (included in the line item Royalty, License and Other Revenues) included in the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2014, was \$1.3 million and \$3.1 million, respectively. The amount of net income/loss from third-party tenants for the three and nine months ended September 30, 2014, was insignificant to the Company's Consolidated Statements 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 are transaction costs incurred in connection with the acquisition of SRCC of \$0.3 million during the nine months ended September 30, 2014. In connection with the purchase of SRCC, 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 lease and the realization of the remaining balance in deferred rent and the reversal of the related asset retirement obligation upon acquisition of the SRCC. \$2.7 million and \$6.1 million of the gain were included in SG&A and Research and Development (R&D) expenses, respectively. The allocation of the

gain to SG&A and R&D is consistent with the Company's allocation practices for facility costs for this previously leased space.

**(6) STOCKHOLDERS' EQUITY**

In March 2014, the Company sold 1.5 million shares of its common stock at a price of \$78.45 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 \$117.5 million from this public offering.



**Table of Contents****BIOMARIN PHARMACEUTICAL INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Continued)****(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)****(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, common stock held by the Company's Nonqualified Deferred Compensation Plan and contingent issuances of common stock related to convertible debt.

The following table sets forth the computation of basic and diluted earnings per common share:

|                                                      | <b>Three Months Ended<br/>September 30,</b> |             | <b>Nine Months Ended<br/>September 30,</b> |              |
|------------------------------------------------------|---------------------------------------------|-------------|--------------------------------------------|--------------|
|                                                      | <b>2014</b>                                 | <b>2013</b> | <b>2014</b>                                | <b>2013</b>  |
| <b>Numerator:</b>                                    |                                             |             |                                            |              |
| Net income (loss), basic                             | \$ 7,445                                    | \$ (53,020) | \$ (64,172)                                | \$ (114,363) |
| Interest expense related to the 2017 Notes           | 156                                         | 0           | 0                                          | 0            |
| Net income (loss), diluted                           | \$ 7,601                                    | \$ (53,020) | \$ (64,172)                                | \$ (114,363) |
| <b>Denominator (in thousands of common shares):</b>  |                                             |             |                                            |              |
| Basic weighted-average shares outstanding            | 147,016                                     | 140,796     | 145,724                                    | 136,102      |
| <b>Effect of dilutive securities:</b>                |                                             |             |                                            |              |
| Options to purchase common stock                     | 9,275                                       | 0           | 0                                          | 0            |
| Common stock issuable under the 2017 Notes           | 2,238                                       | 0           | 0                                          | 0            |
| Unvested restricted stock units (RSUs)               | 642                                         | 0           | 0                                          | 0            |
| Potentially issuable common stock for ESPP purchases | 133                                         | 0           | 0                                          | 0            |
| Fully diluted weighted-average shares                | 159,304                                     | 140,796     | 145,724                                    | 136,102      |
| Basic net income (loss) per common share             | \$ 0.05                                     | \$ (0.38)   | \$ (0.44)                                  | \$ (0.84)    |
| Diluted net income (loss) per common share           | \$ 0.05                                     | \$ (0.38)   | \$ (0.44)                                  | \$ (0.84)    |

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):

|                                                                  | <b>Three Months Ended</b> |               | <b>Nine Months Ended</b> |               |
|------------------------------------------------------------------|---------------------------|---------------|--------------------------|---------------|
|                                                                  | <b>September 30,</b>      |               | <b>September 30,</b>     |               |
|                                                                  | <b>2014</b>               | <b>2013</b>   | <b>2014</b>              | <b>2013</b>   |
| Options to purchase common stock                                 | 3,491                     | 13,421        | 12,780                   | 13,421        |
| Common stock issuable under the 2017 Notes                       | 0                         | 3,846         | 2,238                    | 3,846         |
| Common stock issuable under the 2018 Notes and the 2020 Notes    | 7,966                     | 0             | 7,966                    | 0             |
| Unvested restricted stock units (RSUs)                           | 559                       | 1,235         | 1,216                    | 1,180         |
| Potentially issuable common stock for ESPP purchases             | 0                         | 290           | 135                      | 282           |
| Common stock held by the Nonqualified Deferred Compensation Plan | 224                       | 194           | 224                      | 194           |
| <b>Total number of potentially issuable shares</b>               | <b>12,240</b>             | <b>18,986</b> | <b>24,559</b>            | <b>18,923</b> |

The effect of the 0.75% senior subordinated convertible notes due in 2018 (the 2018 Notes) and the 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 income/loss per 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, the 2018 Notes and the 2020 Notes have no effect on diluted net income/loss per share until the Company's stock price exceeds the conversion price of \$94.15 per share for the Notes.

**Table of Contents****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, 2014 and December 31, 2013. 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, 2014 and December 31, 2013 are summarized in the tables below:

|                                   | <b>Amortized<br/>Cost</b> | <b>Gross<br/>Unrealized<br/>Holding Gains</b> | <b>Gross<br/>Unrealized<br/>Holding Losses</b> | <b>Aggregate<br/>Fair Value at<br/>September 30, 2014</b> |
|-----------------------------------|---------------------------|-----------------------------------------------|------------------------------------------------|-----------------------------------------------------------|
| Certificates of deposit           | \$ 54,342                 | \$ 9                                          | \$ 0                                           | \$ 54,351                                                 |
| Corporate debt securities         | 497,734                   | 334                                           | (862)                                          | 497,206                                                   |
| Commercial paper                  | 19,218                    | 0                                             | 0                                              | 19,218                                                    |
| U.S. Government agency securities | 145,901                   | 23                                            | (159)                                          | 145,765                                                   |
| Greek government-issued bonds     | 50                        | 106                                           | 0                                              | 156                                                       |
| Total                             | \$ 717,245                | \$ 472                                        | \$ (1,021)                                     | \$ 716,696                                                |

|                                   | <b>Amortized<br/>Cost</b> | <b>Gross<br/>Unrealized<br/>Holding Gains</b> | <b>Gross<br/>Unrealized<br/>Holding Losses</b> | <b>Aggregate<br/>Fair Value at<br/>December 31, 2013</b> |
|-----------------------------------|---------------------------|-----------------------------------------------|------------------------------------------------|----------------------------------------------------------|
| Certificates of deposit           | \$ 47,008                 | \$ 2                                          | \$ 0                                           | \$ 47,010                                                |
| Corporate debt securities         | 341,519                   | 313                                           | (423)                                          | 341,409                                                  |
| Commercial paper                  | 86,154                    | 24                                            | 0                                              | 86,178                                                   |
| U.S. Government agency securities | 8,900                     | 1                                             | 0                                              | 8,901                                                    |
| Greek government-issued bonds     | 52                        | 92                                            | 0                                              | 144                                                      |
| Total                             | \$ 483,633                | \$ 432                                        | \$ (423)                                       | \$ 483,642                                               |

The Company has investments in marketable equity securities which are measured using quoted prices in their respective active market that are considered strategic investments. As of September 30, 2014, the fair value of the Company's strategic investments of \$26.6 million included an unrealized gain of \$19.2 million. As of December 31, 2013, the fair value of the Company's strategic investments of \$13.0 million includes an unrealized gain of \$10.1 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:

|                                            | <b>September 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|--------------------------------------------|-------------------------------|------------------------------|
| Maturing in one year or less               | \$ 268,368                    | \$ 215,942                   |
| Maturing after one year through five years | 448,328                       | 267,700                      |
| <b>Total</b>                               | <b>\$ 716,696</b>             | <b>\$ 483,642</b>            |

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, 2014, 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 14 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of the Company's available-for-sale securities.

Table of Contents**BIOMARIN PHARMACEUTICAL INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Continued)****(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)****(9) INTANGIBLE ASSETS**

Intangible assets consisted of the following:

|                                    | September 30,<br>2014 | December 31,<br>2013 |
|------------------------------------|-----------------------|----------------------|
| Intangible assets:                 |                       |                      |
| Finite-lived intangible assets     | \$ 123,785            | \$ 118,242           |
| Indefinite-lived intangible assets | 74,430                | 74,430               |
| Gross intangible assets:           | 198,215               | 192,672              |
| Less: Accumulated amortization     | (38,266)              | (29,525)             |
| Net carrying value                 | \$ 159,949            | \$ 163,147           |

*Indefinite-Lived Intangible Assets*

Indefinite-lived intangible assets consist of in-process research and development (IPR&D) assets related to both early and late stage product candidates purchased in the acquisitions of Huxley Pharmaceuticals Inc. (Huxley), LEAD Therapeutics, Inc. (LEAD), ZyStor Therapeutics, Inc. (ZyStor) and Zacharon Pharmaceuticals, Inc. (Zacharon).

Intangible assets related to IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated research and development 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.

In July 2014, the Company sold the Rare Pediatric Disease Priority Review Voucher (PRV) it received from the U.S. Food and Drug Administration (FDA) in connection with the U.S. approval of VIMIZIM. In exchange for the voucher the Company received \$67.5 million from Regeneron Ireland (Regeneron), an indirect, wholly-owned subsidiary of Regeneron Pharmaceuticals, Inc. The proceeds from the sale of the PRV were recognized as a gain on the sale of intangible asset as the PRV did not have a carrying value on the Company's Condensed Consolidated Balance Sheet at the time of sale.

See Note 10 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013, for additional information related to the Company's Intangible Assets.

**(10) PROPERTY, PLANT AND EQUIPMENT**

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

|                                          | <b>September 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|------------------------------------------|-------------------------------|------------------------------|
| Leasehold improvements                   | \$ 64,629                     | \$ 73,973                    |
| Building and improvements                | 309,255                       | 159,125                      |
| Manufacturing and laboratory equipment   | 114,761                       | 95,126                       |
| Computer hardware and software           | 86,586                        | 74,948                       |
| Furniture and equipment                  | 12,958                        | 12,367                       |
| Land improvements                        | 4,105                         | 0                            |
| Land                                     | 29,357                        | 11,608                       |
| Construction-in-progress                 | 80,982                        | 77,212                       |
|                                          | 702,633                       | 504,359                      |
| Less: Accumulated depreciation           | (215,892)                     | (185,043)                    |
| Total property, plant and equipment, net | \$ 486,741                    | \$ 319,316                   |

Table of Contents**BIOMARIN PHARMACEUTICAL INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Continued)****(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)**

During the nine months ended September 30, 2014, the Company purchased two buildings in Novato, California which were accounted for as an asset purchase. The total purchase price for the buildings was \$20.0 million.

The following table summarizes the allocation of the total purchase price of the buildings:

|                                   | <b>Estimated<br/>Fair Value</b> | <b>Estimated Useful Lives</b> |
|-----------------------------------|---------------------------------|-------------------------------|
| Building and improvements         | \$ 15,836                       | 35 to 45 years                |
| Land                              | 3,216                           |                               |
| Land improvements                 | 919                             | 10 years                      |
| <br>Total identifiable net assets | <br>\$ 19,971                   |                               |

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 was capitalized into inventory, respectively. Depreciation expense for the three and nine months ended September 30, 2013 was \$9.2 million and \$27.3 million, respectively, of which \$2.8 million and \$8.2 million was capitalized into inventory, respectively.

Capitalized interest related to the Company's property, plant and equipment purchases for each of the three and nine months ended September 30, 2014 and 2013 was insignificant.

**(11) SUPPLEMENTAL BALANCE SHEET INFORMATION**

Inventory consisted of the following:

|                     | <b>September 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|---------------------|-------------------------------|------------------------------|
| Raw materials       | \$ 19,946                     | \$ 15,309                    |
| Work-in-process     | 120,477                       | 88,417                       |
| Finished goods      | 64,021                        | 58,879                       |
| <br>Total inventory | <br>\$ 204,444                | <br>\$ 162,605               |

Other Assets consisted of the following:

|                                  | <b>September 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|----------------------------------|-------------------------------|------------------------------|
| Deposits                         | \$ 12,476                     | \$ 7,196                     |
| Escrow balance for SRCC purchase | 0                             | 116,500                      |
| Deferred offering costs          | 12,613                        | 15,374                       |
| Strategic investments            | 26,556                        | 13,000                       |
| Other                            | 9,887                         | 4,101                        |
| <br>Total other assets           | <br>\$ 61,532                 | <br>\$ 156,171               |



**Table of Contents****BIOMARIN PHARMACEUTICAL INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Continued)****(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)**

Accounts payable and accrued liabilities consisted of the following:

|                                                                 | <b>September 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|-----------------------------------------------------------------|-------------------------------|------------------------------|
| Accounts payable                                                | \$ 37,689                     | \$ 36,894                    |
| Accrued accounts payable                                        | 78,063                        | 58,408                       |
| Accrued compensation expense                                    | 40,918                        | 33,496                       |
| Accrued vacation expense                                        | 11,840                        | 10,487                       |
| Current portion of contingent acquisition consideration payable | 0                             | 11,882                       |
| Accrued rebates payable                                         | 13,711                        | 10,429                       |
| Accrued royalties payable                                       | 7,572                         | 5,829                        |
| Value added taxes payable                                       | 4,705                         | 3,603                        |
| Other accrued operating expenses                                | 5,654                         | 4,875                        |
| Current portion of nonqualified deferred compensation liability | 1,372                         | 1,363                        |
| Other                                                           | 5,026                         | 6,005                        |
| Total accounts payable and accrued liabilities                  | \$ 206,550                    | \$ 183,271                   |

**(12) CONVERTIBLE DEBT**

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

|                                                                                                | <b>September 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|------------------------------------------------------------------------------------------------|-------------------------------|------------------------------|
| Convertible Notes due 2020, net of unamortized discount of \$79,836 and \$87,975, respectively | \$ 295,164                    | \$ 287,025                   |
| Convertible Notes due 2018, net of unamortized discount of \$58,839 and \$68,500, respectively | 316,161                       | 306,500                      |
| Convertible Notes due 2017                                                                     | 45,559                        | 62,041                       |
| Total long-term convertible debt, net of unamortized discount                                  | \$ 656,884                    | \$ 655,566                   |

**Fair value of fixed rate convertible debt**

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|                                              |           |                |           |                  |
|----------------------------------------------|-----------|----------------|-----------|------------------|
| Convertible Notes due in 2020 <sup>(1)</sup> | \$        | 412,433        | \$        | 400,879          |
| Convertible Notes due in 2018 <sup>(1)</sup> |           | 400,609        |           | 397,691          |
| Convertible Notes due in 2017 <sup>(1)</sup> |           | 162,807        |           | 213,765          |
| <b>Total</b>                                 | <b>\$</b> | <b>975,849</b> | <b>\$</b> | <b>1,012,335</b> |

(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.

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

|                                                   | <b>Three Months Ended<br/>September 30,</b> |               | <b>Nine Months<br/>Ended<br/>September 30,</b> |                 |
|---------------------------------------------------|---------------------------------------------|---------------|------------------------------------------------|-----------------|
|                                                   | <b>2014</b>                                 | <b>2013</b>   | <b>2014</b>                                    | <b>2013</b>     |
| Coupon interest                                   | \$ 2,278                                    | \$ 463        | \$ 7,140                                       | \$ 2,502        |
| Amortization of issuance costs                    | 828                                         | 63            | 2,505                                          | 352             |
| Accretion of debt discount                        | 6,012                                       | 0             | 17,800                                         | 0               |
| <b>Total interest expense on convertible debt</b> | <b>\$ 9,118</b>                             | <b>\$ 526</b> | <b>\$ 27,445</b>                               | <b>\$ 2,854</b> |

During the nine months ended September 30, 2014, the Company entered into two separate agreements with an existing holder of its senior subordinated convertible notes due in 2017 (the 2017 Notes) pursuant to which such holder 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 aggregate, of which \$0.7 million was recognized in total as Debt Conversion Expense on the Condensed Consolidated Statement of Comprehensive Income (Loss) for the nine months ended September 30, 2014.

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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)**

See Note 5 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013, for additional information related to the Company's Convertible Debt.

**(13) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES**

***Foreign Currency Exchange Rate Exposure***

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 Naglazyme product revenues, Aldurazyme royalty revenues, operating expenses and net 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. Details of the specific instruments used by the Company to hedge its exposure to foreign currency exchange rate fluctuations are discussed below. See Note 14 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of forward foreign currency exchange contracts.

At September 30, 2014, the Company had 80 forward foreign currency exchange contracts outstanding to sell a total of 106.6 million Euros with expiration dates ranging from October 2014 through February 2017. These hedges were entered into in order to protect against the fluctuations in revenue associated with Euro denominated Naglazyme and Aldurazyme sales. 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 denominated in Euros related to changes in foreign currency exchange rates.

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, 2014, the Company had one outstanding forward foreign currency exchange contract to buy 44.1 million Euros, which was not designated as a hedge for accounting purposes and matures on October 31, 2014.

The maximum length of time over which the Company is hedging its exposure to the reduction in value of forecasted foreign currency cash flows through forward foreign currency exchange contracts is through February 2017. Over the next twelve months, the Company expects to reclassify \$6.1 million from accumulated other comprehensive income to

earnings as the forecasted revenue transactions occur.

Table of Contents**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 fair value carrying amounts of the Company's derivative instruments were as follows:

|                                                           | <b>Asset Derivatives</b>      |                   | <b>Liability Derivatives</b>             |                   |
|-----------------------------------------------------------|-------------------------------|-------------------|------------------------------------------|-------------------|
|                                                           | <b>September 30, 2014</b>     |                   | <b>September 30, 2014</b>                |                   |
|                                                           | <b>Balance Sheet Location</b> | <b>Fair Value</b> | <b>Balance Sheet Location</b>            | <b>Fair Value</b> |
| <b>Derivatives designated as hedging instruments:</b>     |                               |                   |                                          |                   |
| Forward foreign currency exchange contracts               | Other current assets          | \$ 6,040          | Accounts payable and accrued liabilities | \$ 0              |
| Forward foreign currency exchange contracts               | Other assets                  | 4,760             | Other long-term liabilities              | 0                 |
| Total                                                     |                               | \$ 10,800         |                                          | \$ 0              |
| <b>Derivatives not designated as hedging instruments:</b> |                               |                   |                                          |                   |
| Forward foreign currency exchange contracts               | Other current assets          | \$ 0              | Accounts payable and accrued liabilities | \$ 94             |
| Total                                                     |                               | 0                 |                                          | 94                |
| Total value of derivative contracts                       |                               | \$ 10,800         |                                          | \$ 94             |

|                                                       | <b>Asset Derivatives</b>      |                   | <b>Liability Derivatives</b>             |                   |
|-------------------------------------------------------|-------------------------------|-------------------|------------------------------------------|-------------------|
|                                                       | <b>December 31, 2013</b>      |                   | <b>December 31, 2013</b>                 |                   |
|                                                       | <b>Balance Sheet Location</b> | <b>Fair Value</b> | <b>Balance Sheet Location</b>            | <b>Fair Value</b> |
| <b>Derivatives designated as hedging instruments:</b> |                               |                   |                                          |                   |
| Forward foreign currency exchange contracts           | Other current assets          | \$ 0              | Accounts payable and accrued liabilities | \$ 2,186          |

|                                             |              |      |                             |          |
|---------------------------------------------|--------------|------|-----------------------------|----------|
| Forward foreign currency exchange contracts | Other assets | 0    | Other long-term liabilities | 0        |
| Total                                       |              | \$ 0 |                             | \$ 2,186 |

**Derivatives not designated as hedging instruments:**

|                                             |                      |       |                                          |      |
|---------------------------------------------|----------------------|-------|------------------------------------------|------|
| Forward foreign currency exchange contracts | Other current assets | \$ 59 | Accounts payable and accrued liabilities | \$ 0 |
| Total                                       |                      | 59    |                                          | 0    |

|                                     |       |          |
|-------------------------------------|-------|----------|
| Total value of derivative contracts | \$ 59 | \$ 2,186 |
|-------------------------------------|-------|----------|

The effect of the Company's derivative instruments on the Condensed Consolidated Financial Statements for the three and nine months ended September 30, 2014 and 2013 was as follows:

|                                                                               | Forward Foreign Currency Exchange Contracts |            |                   |            |
|-------------------------------------------------------------------------------|---------------------------------------------|------------|-------------------|------------|
|                                                                               | Three Months Ended                          |            | Nine Months Ended |            |
|                                                                               | September 30,                               |            | September 30,     |            |
|                                                                               | 2014                                        | 2013       | 2014              | 2013       |
| <b>Derivatives Designated as Hedging Instruments:</b>                         |                                             |            |                   |            |
| Net gain (loss) recognized in Other Comprehensive Income (OCI) <sup>(1)</sup> | \$ 6,247                                    | \$ (2,094) | \$ 9,395          | \$ (1,844) |
| Net gain (loss) reclassified from accumulated OCI into income <sup>(2)</sup>  | 359                                         | 65         | (662)             | 619        |
| Net gain (loss) recognized in income <sup>(3)</sup>                           | (179)                                       | 10         | (499)             | 214        |
| <b>Derivatives Not Designated as Hedging Instruments:</b>                     |                                             |            |                   |            |
| Net gain (loss) recognized in income <sup>(4)</sup>                           | \$ 4,365                                    | \$ (1,437) | \$ 4,861          | \$ (1,129) |

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

(2) Effective portion classified as net product revenue.

(3) Ineffective portion and amount excluded from effectiveness testing classified as selling, general and administrative expense.

(4) Classified as selling, general and administrative expense.

At September 30, 2014 and December 31, 2013, accumulated other comprehensive income before taxes associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a gain of \$11.3 million and a loss of \$2.4 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.



**Table of Contents****BIOMARIN PHARMACEUTICAL INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Continued)****(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)****(14) 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.

| <b>Fair Value Measurements at September 30, 2014</b> |                                                                                              |                                                              |                                                              |                   |
|------------------------------------------------------|----------------------------------------------------------------------------------------------|--------------------------------------------------------------|--------------------------------------------------------------|-------------------|
|                                                      | <b>Quoted Price in<br/>Active<br/>Markets<br/>for<br/>Identical<br/>Assets<br/>(Level 1)</b> | <b>Significant Other<br/>Observable Inputs<br/>(Level 2)</b> | <b>Significant<br/>Unobservable<br/>Inputs<br/>(Level 3)</b> | <b>Total</b>      |
| <b>Assets:</b>                                       |                                                                                              |                                                              |                                                              |                   |
| Cash and cash equivalents:                           |                                                                                              |                                                              |                                                              |                   |
| Overnight deposits                                   | \$ 302,655                                                                                   | \$ 0                                                         | \$ 0                                                         | \$ 302,655        |
| Money market instruments                             | 0                                                                                            | 95,350                                                       | 0                                                            | 95,350            |
| <b>Total cash and cash equivalents</b>               | <b>\$ 302,655</b>                                                                            | <b>\$ 95,350</b>                                             | <b>\$ 0</b>                                                  | <b>\$ 398,005</b> |
| Available-for-sale securities:                       |                                                                                              |                                                              |                                                              |                   |
| Short-term:                                          |                                                                                              |                                                              |                                                              |                   |
| Certificates of deposit                              | \$ 0                                                                                         | \$ 32,302                                                    | \$ 0                                                         | \$ 32,302         |
| Corporate debt securities                            | 0                                                                                            | 187,409                                                      | 0                                                            | 187,409           |
| Commercial paper                                     | 0                                                                                            | 19,218                                                       | 0                                                            | 19,218            |
| U.S. Government agency securities                    | 0                                                                                            | 29,439                                                       | 0                                                            | 29,439            |
| Long-term:                                           |                                                                                              |                                                              |                                                              |                   |
| Certificates of deposit                              | 0                                                                                            | 22,049                                                       | 0                                                            | 22,049            |
| Corporate debt securities                            | 0                                                                                            | 309,797                                                      | 0                                                            | 309,797           |
| U.S. Government agency securities                    | 0                                                                                            | 116,326                                                      | 0                                                            | 116,326           |
| Greek government-issued bonds                        | 0                                                                                            | 156                                                          | 0                                                            | 156               |
| <b>Total available-for-sale securities</b>           | <b>\$ 0</b>                                                                                  | <b>\$ 716,696</b>                                            | <b>\$ 0</b>                                                  | <b>\$ 716,696</b> |
| Other Current Assets:                                |                                                                                              |                                                              |                                                              |                   |
| Nonqualified Deferred Compensation Plan assets       | \$ 0                                                                                         | \$ 342                                                       | \$ 0                                                         | \$ 342            |
|                                                      | 0                                                                                            | 6,040                                                        | 0                                                            | 6,040             |



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Forward foreign currency exchange contract  
assets <sup>(1)</sup>

|                                       |   |       |   |       |
|---------------------------------------|---|-------|---|-------|
| Restricted investments <sup>(2)</sup> | 0 | 2,352 | 0 | 2,352 |
|---------------------------------------|---|-------|---|-------|

|                            |      |          |      |          |
|----------------------------|------|----------|------|----------|
| Total other current assets | \$ 0 | \$ 8,734 | \$ 0 | \$ 8,734 |
|----------------------------|------|----------|------|----------|

**Other Assets:**

|                                                |      |          |      |          |
|------------------------------------------------|------|----------|------|----------|
| Nonqualified Deferred Compensation Plan assets | \$ 0 | \$ 4,890 | \$ 0 | \$ 4,890 |
|------------------------------------------------|------|----------|------|----------|

|                                                                     |   |       |   |       |
|---------------------------------------------------------------------|---|-------|---|-------|
| Forward foreign currency exchange contract<br>assets <sup>(1)</sup> | 0 | 4,760 | 0 | 4,760 |
|---------------------------------------------------------------------|---|-------|---|-------|

|                                     |        |   |   |        |
|-------------------------------------|--------|---|---|--------|
| Strategic investment <sup>(3)</sup> | 26,556 | 0 | 0 | 26,556 |
|-------------------------------------|--------|---|---|--------|

|                    |           |          |      |           |
|--------------------|-----------|----------|------|-----------|
| Total other assets | \$ 26,556 | \$ 9,650 | \$ 0 | \$ 36,206 |
|--------------------|-----------|----------|------|-----------|

|              |            |            |      |              |
|--------------|------------|------------|------|--------------|
| Total assets | \$ 329,211 | \$ 830,430 | \$ 0 | \$ 1,159,641 |
|--------------|------------|------------|------|--------------|

**Liabilities:**

**Current Liabilities:**

|                                                      |          |        |      |          |
|------------------------------------------------------|----------|--------|------|----------|
| Nonqualified Deferred Compensation Plan<br>liability | \$ 1,030 | \$ 342 | \$ 0 | \$ 1,372 |
|------------------------------------------------------|----------|--------|------|----------|

|                                                                        |   |    |   |    |
|------------------------------------------------------------------------|---|----|---|----|
| Forward foreign currency exchange contract<br>liability <sup>(1)</sup> | 0 | 94 | 0 | 94 |
|------------------------------------------------------------------------|---|----|---|----|

|                                   |   |   |    |    |
|-----------------------------------|---|---|----|----|
| Asset retirement obligation (ARO) | 0 | 0 | 29 | 29 |
|-----------------------------------|---|---|----|----|

|                           |          |        |       |          |
|---------------------------|----------|--------|-------|----------|
| Total current liabilities | \$ 1,030 | \$ 436 | \$ 29 | \$ 1,495 |
|---------------------------|----------|--------|-------|----------|

**Other long-term liabilities:**

|                                                      |           |          |      |           |
|------------------------------------------------------|-----------|----------|------|-----------|
| Nonqualified Deferred Compensation Plan<br>liability | \$ 15,096 | \$ 4,890 | \$ 0 | \$ 19,986 |
|------------------------------------------------------|-----------|----------|------|-----------|

|                                              |   |   |        |        |
|----------------------------------------------|---|---|--------|--------|
| Contingent acquisition consideration payable | 0 | 0 | 42,297 | 42,297 |
|----------------------------------------------|---|---|--------|--------|

|                             |   |   |       |       |
|-----------------------------|---|---|-------|-------|
| Asset retirement obligation | 0 | 0 | 3,138 | 3,138 |
|-----------------------------|---|---|-------|-------|

|                                   |           |          |           |           |
|-----------------------------------|-----------|----------|-----------|-----------|
| Total other long-term liabilities | \$ 15,096 | \$ 4,890 | \$ 45,435 | \$ 65,421 |
|-----------------------------------|-----------|----------|-----------|-----------|

|                   |           |          |           |           |
|-------------------|-----------|----------|-----------|-----------|
| Total liabilities | \$ 16,126 | \$ 5,326 | \$ 45,464 | \$ 66,916 |
|-------------------|-----------|----------|-----------|-----------|

Table of Contents**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, 2013**

|  | <b>Quoted Price in<br/>Active<br/>Markets<br/>For<br/>Identical<br/>Assets<br/>(Level 1)</b> | <b>Significant Other<br/>Observable<br/>Inputs<br/>(Level 2)</b> | <b>Significant<br/>Unobservable<br/>Inputs<br/>(Level 3)</b> | <b>Total</b> |
|--|----------------------------------------------------------------------------------------------|------------------------------------------------------------------|--------------------------------------------------------------|--------------|
|--|----------------------------------------------------------------------------------------------|------------------------------------------------------------------|--------------------------------------------------------------|--------------|

**Assets:**

## Cash and cash equivalents:

|                          |            |         |      |            |
|--------------------------|------------|---------|------|------------|
| Overnight deposits       | \$ 156,228 | \$ 0    | \$ 0 | \$ 156,228 |
| Money market instruments | 0          | 412,553 | 0    | 412,553    |

|                                        |                   |                   |             |                   |
|----------------------------------------|-------------------|-------------------|-------------|-------------------|
| <b>Total cash and cash equivalents</b> | <b>\$ 156,228</b> | <b>\$ 412,553</b> | <b>\$ 0</b> | <b>\$ 568,781</b> |
|----------------------------------------|-------------------|-------------------|-------------|-------------------|

## Available-for-sale securities:

## Short-term:

|                           |      |           |      |           |
|---------------------------|------|-----------|------|-----------|
| Certificates of deposit   | \$ 0 | \$ 30,513 | \$ 0 | \$ 30,513 |
| Corporate debt securities | 0    | 99,251    | 0    | 99,251    |
| Commercial paper          | 0    | 86,178    | 0    | 86,178    |

## Long-term:

|                                   |   |         |   |         |
|-----------------------------------|---|---------|---|---------|
| Certificates of deposit           | 0 | 16,497  | 0 | 16,497  |
| Corporate debt securities         | 0 | 242,158 | 0 | 242,158 |
| U.S. Government agency securities | 0 | 8,901   | 0 | 8,901   |
| Greek government-issued bonds     | 0 | 144     | 0 | 144     |

|                                            |             |                   |             |                   |
|--------------------------------------------|-------------|-------------------|-------------|-------------------|
| <b>Total available-for-sale securities</b> | <b>\$ 0</b> | <b>\$ 483,642</b> | <b>\$ 0</b> | <b>\$ 483,642</b> |
|--------------------------------------------|-------------|-------------------|-------------|-------------------|

## Other Current Assets:

|                                                                  |      |        |      |        |
|------------------------------------------------------------------|------|--------|------|--------|
| Nonqualified Deferred Compensation Plan assets                   | \$ 0 | \$ 136 | \$ 0 | \$ 136 |
| Forward foreign currency exchange contract assets <sup>(1)</sup> | 0    | 59     | 0    | 59     |
| Restricted investments <sup>(2)</sup>                            | 0    | 5,670  | 0    | 5,670  |

|                                   |             |                 |             |                 |
|-----------------------------------|-------------|-----------------|-------------|-----------------|
| <b>Total other current assets</b> | <b>\$ 0</b> | <b>\$ 5,865</b> | <b>\$ 0</b> | <b>\$ 5,865</b> |
|-----------------------------------|-------------|-----------------|-------------|-----------------|

## Other Assets:

|                                                |        |          |      |          |
|------------------------------------------------|--------|----------|------|----------|
| Nonqualified Deferred Compensation Plan assets | \$ 0   | \$ 3,459 | \$ 0 | \$ 3,459 |
| Restricted investments <sup>(2)</sup>          | 0      | 412      | 0    | 412      |
| Strategic investment <sup>(3)</sup>            | 13,000 | 0        | 0    | 13,000   |

|                     |                   |                   |             |                     |
|---------------------|-------------------|-------------------|-------------|---------------------|
| Total other assets  | \$ 13,000         | \$ 3,871          | \$ 0        | \$ 16,871           |
| <b>Total assets</b> | <b>\$ 169,228</b> | <b>\$ 905,931</b> | <b>\$ 0</b> | <b>\$ 1,075,159</b> |

**Liabilities:****Current Liabilities:**

|                                                                     |                 |                 |                  |                  |
|---------------------------------------------------------------------|-----------------|-----------------|------------------|------------------|
| Nonqualified Deferred Compensation Plan liability                   | \$ 1,227        | \$ 136          | \$ 0             | \$ 1,363         |
| Forward foreign currency exchange contract liability <sup>(1)</sup> | 0               | 2,186           | 0                | 2,186            |
| Contingent acquisition consideration payable                        | 0               | 0               | 11,882           | 11,882           |
| <b>Total current liabilities</b>                                    | <b>\$ 1,227</b> | <b>\$ 2,322</b> | <b>\$ 11,882</b> | <b>\$ 15,431</b> |

**Other long-term liabilities:**

|                                                   |                  |                 |                  |                  |
|---------------------------------------------------|------------------|-----------------|------------------|------------------|
| Nonqualified Deferred Compensation Plan liability | \$ 12,345        | \$ 3,459        | \$ 0             | \$ 15,804        |
| Contingent acquisition consideration payable      | 0                | 0               | 30,790           | 30,790           |
| Asset retirement obligation                       | 0                | 0               | 4,122            | 4,122            |
| <b>Total other long-term liabilities</b>          | <b>\$ 12,345</b> | <b>\$ 3,459</b> | <b>\$ 34,912</b> | <b>\$ 50,716</b> |
| <b>Total liabilities</b>                          | <b>\$ 13,572</b> | <b>\$ 5,781</b> | <b>\$ 46,794</b> | <b>\$ 66,147</b> |

- (1) See Note 13 to these Condensed Consolidated Financial Statements for further information regarding the derivative instruments.
- (2) The restricted investments at September 30, 2014 secure the Company's irrevocable standby letter of credit obtained in connection with certain commercial agreements. The restricted investments at December 31, 2013 secure the Company's irrevocable standby letter of credit obtained in connection with the Company's SRCC lease and certain commercial agreements.
- (3) 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.

**Table of Contents****BIOMARIN PHARMACEUTICAL INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Continued)****(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)**

There were no transfers between levels during the nine months ended September 30, 2014.

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, 2013             | \$ 42,672 |
| Changes in the fair value of the contingent acquisition consideration payable | 12,625    |
| Milestone payments to former ZyStor shareholders                              | (13,000)  |
| Contingent acquisition consideration payable at September 30, 2014            | \$ 42,297 |

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, 2013                              | \$ 4,122 |
| Accretion expense                                                              | 86       |
| Release of ARO accruals related to purchases of previously leased office space | (1,041)  |
| Asset retirement obligations at September 30, 2014                             | \$ 3,167 |

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.

Table of Contents**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) 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 and the 2012 Inducement Plan, which expired in May 2013. The Company's stock-based compensation plans are administered by the Compensation Committee of the Board of Directors, 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 award. 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, 2013, 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, but none were identified that had distinctly different exercise patterns as of September 30, 2014. 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 and the Share Incentive Plan were as follows:

|                         | <b>Three Months Ended</b> |      | <b>September 30,</b> |  | <b>Nine Months Ended</b> |      | <b>September 30,</b> |           |
|-------------------------|---------------------------|------|----------------------|--|--------------------------|------|----------------------|-----------|
|                         | <b>2014</b>               |      | <b>2013</b>          |  | <b>2014</b>              |      | <b>2013</b>          |           |
| Expected volatility     | 44                        | 45%  | 44%                  |  | 44                       | 45%  | 44                   | 45%       |
| Dividend yield          | 0.0%                      |      | 0.0%                 |  | 0.0%                     |      | 0.0%                 |           |
| Expected life           | 6.9 years                 |      | 6.8 years            |  | 6.9 years                |      | 6.6                  | 6.8 years |
| Risk-free interest rate | 2.0                       | 2.2% | 2.2%                 |  | 2.0                      | 2.3% | 1.0                  | 2.4%      |

During the nine months ended September 30, 2014, the Company granted 1,177,376 options with a weighted average fair value of \$30.90 per option.

The Company did not issue any new stock purchase rights under the ESPP during the three months ended September 30, 2014.

***Restricted Stock Unit Awards with Service-Based Vesting Conditions***

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, 2014, the Company granted 835,642 RSUs with a weighted average fair market value of \$63.58 per share.

**Table of Contents****BIOMARIN PHARMACEUTICAL INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Continued)****(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)*****Restricted Stock Unit Awards with Performance and Market-Based Vesting Conditions***

Pursuant to the approval of the Board of Directors, the Company granted RSU awards with performance and market-based vesting conditions during 2012 and 2011 to certain executive officers. As of September 30, 2014, these awards provide for a base award of 860,000 RSUs (the Base RSUs), with a weighted-average grant date fair value of \$34.66. The number of RSUs that could potentially vest from the Base RSUs granted is contingent upon achievement of specific performance goals and will be multiplied by the Total Shareholder Return multiplier which could range from 75% to 125% to determine the number of earned RSUs.

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 2013, management concluded that regulatory approval of VIMIZIM was probable and the Company began recognizing compensation expense related to the performance based RSUs allocated to this performance goal. The Company recognized compensation expense of \$0.7 million and \$1.6 million for these awards during three and nine months ended September 30, 2014, respectively. For the three and nine months ended September 30, 2013, the Company did not recognize any expense related to these awards because the Company's management had not yet determined the goals were probable of achievement.

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

|                                        | <b>Three Months Ended<br/>September 30,</b> |             | <b>Nine Months Ended<br/>September 30,</b> |             |
|----------------------------------------|---------------------------------------------|-------------|--------------------------------------------|-------------|
|                                        | <b>2014</b>                                 | <b>2013</b> | <b>2014</b>                                | <b>2013</b> |
| Cost of sales                          | \$ 1,180                                    | \$ 1,489    | \$ 3,807                                   | \$ 3,663    |
| Research and development               | 8,279                                       | 7,116       | 22,300                                     | 18,821      |
| Selling, general and administrative    | 10,545                                      | 7,600       | 27,452                                     | 19,214      |
| Total stock-based compensation expense | \$ 20,004                                   | \$ 16,205   | \$ 53,559                                  | \$ 41,698   |

Stock-based compensation of \$5.7 million and \$4.2 million was capitalized into inventory for the nine months ended September 30, 2014 and 2013, respectively. Capitalized stock-based compensation is recognized as cost of sales when the related product is sold.

**(16) COMPREHENSIVE INCOME (LOSS)**

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income/(Loss) (AOI) and their effect on the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) for



the three and nine months ended September 30, 2014 and 2013.

| Details about AOCI Components               | Amount Reclassified From AOCI (Gain) Loss |                                       |                                      |                                      | Condensed Consolidated Statement of Comprehensive Loss Classification |
|---------------------------------------------|-------------------------------------------|---------------------------------------|--------------------------------------|--------------------------------------|-----------------------------------------------------------------------|
|                                             | Three Months Ended September 30, 2014     | Three Months Ended September 30, 2013 | Nine Months Ended September 30, 2014 | Nine Months Ended September 30, 2013 |                                                                       |
| (Gains) Losses on cash flow hedges:         |                                           |                                       |                                      |                                      |                                                                       |
| Forward foreign currency exchange contracts | \$ (562)                                  | \$ (101)                              | \$ 1,035                             | \$ (928)                             | Net product revenues                                                  |
| Forward foreign currency exchange contracts | 0                                         | 0                                     | 0                                    | (40)                                 | Selling, general and administrative                                   |
| Income tax effect of the above items        | 203                                       | 36                                    | (373)                                | 349                                  | Provision for (benefit from) income taxes                             |
|                                             | \$ (359)                                  | \$ (65)                               | \$ 662                               | \$ (619)                             | Net income (loss)                                                     |

Table of Contents**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 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, 2014 and 2013.

|                                                                   | <b>Three Months Ended<br/>September 30, 2014</b> |                                      |                              |
|-------------------------------------------------------------------|--------------------------------------------------|--------------------------------------|------------------------------|
|                                                                   | <b>Before<br/>Tax<br/>Amount</b>                 | <b>Tax<br/>(Expense)<br/>Benefit</b> | <b>Net-of-Tax<br/>Amount</b> |
| AOCI balance at June 30, 2014                                     | \$ 15,491                                        | \$ (5,550)                           | \$ 9,941                     |
| Foreign currency translation adjustment                           | (3)                                              | 0                                    | (3)                          |
| Unrealized gain on available-for-sale securities:                 |                                                  |                                      |                              |
| Unrealized holding gains                                          | 4,138                                            | (1,493)                              | 2,645                        |
| Less: reclassification adjustment for gain realized in net loss   | 0                                                | 0                                    | 0                            |
| 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 gain 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                    |

|  | <b>Nine Months Ended<br/>September 30, 2014</b> |                                      |                              |
|--|-------------------------------------------------|--------------------------------------|------------------------------|
|  | <b>Before<br/>Tax<br/>Amount</b>                | <b>Tax<br/>(Expense)<br/>Benefit</b> | <b>Net-of-Tax<br/>Amount</b> |

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|                                                                 |           |             |           |
|-----------------------------------------------------------------|-----------|-------------|-----------|
| AOCI balance at December 31, 2013                               | \$ 7,757  | \$ (2,739)  | \$ 5,018  |
| Foreign currency translation adjustment                         | (36)      | 0           | (36)      |
| Unrealized gain on available-for-sale securities:               |           |             |           |
| Unrealized holding gains                                        | 8,579     | (3,105)     | 5,474     |
| Less: reclassification adjustment for gain realized in net loss | 0         | 0           | 0         |
| Net unrealized holding gain                                     | 8,579     | (3,105)     | 5,474     |
| Net unrealized holding gain on cash flow hedges:                |           |             |           |
| Unrealized holding gain                                         | 14,691    | (5,296)     | 9,395     |
| Less: reclassification adjustment for gain realized in net loss | (1,035)   | 373         | (662)     |
| Net unrealized holding gain                                     | 13,656    | (4,923)     | 8,733     |
| Other comprehensive income                                      | 22,199    | (8,028)     | 14,171    |
| AOCI balance at September 30, 2014                              | \$ 29,956 | \$ (10,767) | \$ 19,189 |

Table of Contents**BIOMARIN PHARMACEUTICAL INC.****NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Continued)****(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)**

|                                                                 | <b>Three Months Ended<br/>September 30, 2013</b> |                                      |                              |
|-----------------------------------------------------------------|--------------------------------------------------|--------------------------------------|------------------------------|
|                                                                 | <b>Before<br/>Tax<br/>Amount</b>                 | <b>Tax<br/>(Expense)<br/>Benefit</b> | <b>Net-of-Tax<br/>Amount</b> |
| AOCI balance at June 30, 2013                                   | \$ 3,801                                         | \$ (1,360)                           | \$ 2,441                     |
| Foreign currency translation adjustment                         | 1,693                                            | 0                                    | 1,639                        |
| Unrealized gain on available-for-sale securities:               |                                                  |                                      |                              |
| Unrealized holding gains                                        | 14,664                                           | (5,296)                              | 9,368                        |
| Less: reclassification adjustment for gain realized in net loss | 0                                                | 0                                    | 0                            |
| Net unrealized holding gain                                     | 14,664                                           | (5,296)                              | 9,368                        |
| Net unrealized holding (loss) on cash flow hedges:              |                                                  |                                      |                              |
| Unrealized holding (loss)                                       | (3,278)                                          | 1,184                                | (2,094)                      |
| Less: reclassification adjustment for gain realized in net loss | 101                                              | (36)                                 | 65                           |
| Net unrealized holding (loss)                                   | (3,177)                                          | 1,148                                | (2,029)                      |
| Other comprehensive income                                      | 13,180                                           | (4,148)                              | 9,032                        |
| AOCI balance at September 30, 2013                              | \$ 16,981                                        | \$ (5,508)                           | \$ 11,473                    |

|                                                   | <b>Nine Months Ended<br/>September 30, 2013</b> |                                      |                              |
|---------------------------------------------------|-------------------------------------------------|--------------------------------------|------------------------------|
|                                                   | <b>Before<br/>Tax<br/>Amount</b>                | <b>Tax<br/>(Expense)<br/>Benefit</b> | <b>Net-of-Tax<br/>Amount</b> |
| AOCI balance at December 31, 2012                 | \$ (222)                                        | \$ 20                                | \$ (202)                     |
| Foreign currency translation adjustment           | 1,893                                           | 0                                    | 1,893                        |
| Unrealized gain on available-for-sale securities: |                                                 |                                      |                              |
| Unrealized holding gains                          | 17,228                                          | (6,221)                              | 11,007                       |

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|                                                                 |           |            |           |
|-----------------------------------------------------------------|-----------|------------|-----------|
| Less: reclassification adjustment for gain realized in net loss | 0         | 0          | 0         |
| Net unrealized holding gain                                     | 17,228    | (6,221)    | 11,007    |
| Net unrealized holding (loss) on cash flow hedges:              |           |            |           |
| Unrealized holding (loss)                                       | (2,886)   | 1,042      | (1,844)   |
| Less: reclassification adjustment for gain realized in net loss | 968       | (349)      | 619       |
| Net unrealized holding (loss)                                   | (1,918)   | 693        | (1,225)   |
| Other comprehensive income                                      | 17,203    | (5,528)    | 11,675    |
| AOCI balance at September 30, 2013                              | \$ 16,981 | \$ (5,508) | \$ 11,473 |

Table of Contents**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) 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 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 and the headquarters for Genzyme Corporation (Genzyme) 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.

|                           | <b>Three Months Ended<br/>September 30,</b> |             | <b>Nine Months Ended<br/>September 30,</b> |             |
|---------------------------|---------------------------------------------|-------------|--------------------------------------------|-------------|
|                           | <b>2014</b>                                 | <b>2013</b> | <b>2014</b>                                | <b>2013</b> |
| Region:                   |                                             |             |                                            |             |
| United States             | 55%                                         | 54%         | 50%                                        | 51%         |
| Europe                    | 22%                                         | 21%         | 19%                                        | 21%         |
| Latin America             | 8%                                          | 8%          | 15%                                        | 12%         |
| Rest of world             | 15%                                         | 17%         | 16%                                        | 16%         |
| 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 four largest customers.

|                           | <b>Three Months Ended<br/>September 30,</b> |             | <b>Nine Months Ended<br/>September 30,</b> |             |
|---------------------------|---------------------------------------------|-------------|--------------------------------------------|-------------|
|                           | <b>2014</b>                                 | <b>2013</b> | <b>2014</b>                                | <b>2013</b> |
| Customer A                | 17%                                         | 15%         | 15%                                        | 15%         |
| Customer B <sup>(1)</sup> | 13%                                         | 17%         | 13%                                        | 15%         |
| Customer C                | 4%                                          | 4%          | 11%                                        | 8%          |
| Customer D                | 13%                                         | 11%         | 11%                                        | 11%         |
| Total                     | 47%                                         | 47%         | 50%                                        | 49%         |

- (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.

The accounts receivable balances at September 30, 2014 and December 31, 2013 were comprised of amounts due from customers for net product sales of VIMIZIM, Naglazyme, Kuvan and Firdapse and Aldurazyme product transfer and royalty revenues. On a consolidated basis, the Company's two largest customers accounted for 36% and 19% of the September 30, 2014 accounts receivable balance, respectively, compared to December 31, 2013 when the two largest customers accounted for 45% and 15% of the accounts receivable balance, respectively. As of September 30, 2014 and December 31, 2013, accounts receivable for the Company's largest customer balance included \$21.7 million and \$26.3 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.

**Table of Contents****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 European Union (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. The Company's net product revenues for these countries was 6% and 4%, for the three and nine months ended September 30, 2014, respectively. Additionally, approximately 9% of the Company's outstanding accounts receivable at September 30, 2014 related to such countries.

As of September 30, 2014, the Company's accounts receivable in certain European countries, specifically Greece, Italy, Portugal, Spain and Russia, totaled approximately \$10.6 million, of which \$0.7 million were greater than 90 days past due, \$0.1 million were greater than 180 days past due and \$0.1 million were greater than 365 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.

**(18) 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 our products have similar economic and other characteristics, including the nature of the products and production processes, type of customers, distribution methods and regulatory environment.

|                                 | <b>Three Months Ended<br/>September 30,</b> |             | <b>Nine Months Ended<br/>September 30,</b> |             |
|---------------------------------|---------------------------------------------|-------------|--------------------------------------------|-------------|
|                                 | <b>2014</b>                                 | <b>2013</b> | <b>2014</b>                                | <b>2013</b> |
| Net product revenue by product: |                                             |             |                                            |             |
| VIMIZIM                         | \$ 25,239                                   | \$ 11       | \$ 40,389                                  | \$ 11       |
| Naglazyme                       | 67,511                                      | 63,263      | 245,954                                    | 202,520     |
| Kuvan                           | 53,438                                      | 43,553      | 145,578                                    | 122,071     |



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|                           |            |            |            |            |
|---------------------------|------------|------------|------------|------------|
| Aldurazyme                | 22,553     | 23,427     | 64,727     | 57,683     |
| Firdapse                  | 4,675      | 4,076      | 14,016     | 11,789     |
| Total net product revenue | \$ 173,416 | \$ 134,330 | \$ 510,664 | \$ 394,074 |

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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)**

**(19) COMMITMENTS AND CONTINGENCIES**

The Company is also subject to contingent payments totaling approximately \$561.9 million as of September 30, 2014 which are due upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, \$57.9 million relates to programs that are no longer being developed.

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, 2014, these commitments for the next five years were approximately \$46.0 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.

**Table of Contents****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, 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, 2013. 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.

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

|                                                                    | <b>Three Months Ended</b> |             | <b>Nine Months Ended</b> |             |
|--------------------------------------------------------------------|---------------------------|-------------|--------------------------|-------------|
|                                                                    | <b>September 30,</b>      |             | <b>September 30,</b>     |             |
|                                                                    | <b>2014</b>               | <b>2013</b> | <b>2014</b>              | <b>2013</b> |
| Total net product revenues                                         | \$ 173.4                  | \$ 134.3    | \$ 510.7                 | \$ 394.1    |
| Cost of sales                                                      | 29.9                      | 28.1        | 83.9                     | 71.1        |
| Research and development expense                                   | 125.7                     | 88.1        | 319.6                    | 257.5       |
| Selling, general and administrative expense                        | 74.6                      | 61.8        | 202.5                    | 163.5       |
| Intangible asset amortization and contingent consideration expense | 2.9                       | 9.6         | 15.8                     | 13.2        |
| Net income (loss)                                                  | 7.4                       | (53.0)      | (64.2)                   | (114.4)     |
| Stock-based compensation expense                                   | 20.0                      | 16.2        | 53.6                     | 41.7        |

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

Our product portfolio is comprised of five approved products and multiple investigational product candidates. Our approved products are VIMIZIM (elosulfase alpha), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

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

VIMIZIM, a treatment for mucopolysaccharidosis Type IVA or Morquio Syndrome Type A, a lysosomal storage disorder, received marketing approval in the U.S. in February 2014 and in the European Union (the EU) in April 2014. We immediately began marketing VIMIZIM in each of these markets using our existing sales force and commercial organization and completed our first commercial sale in the U.S. and the EU in February 2014 and April 2014, respectively. VIMIZIM net product revenues for the three and nine months ended September 30, 2014 totaled \$25.2 million and \$40.4 million, respectively.

Naglazyme, a recombinant form of N-acetylgalactosamine 4-sulfatase indicated for patients with mucopolysaccharidosis VI (MPS VI), a debilitating life-threatening genetic disease for which no other drug treatment currently exists and which is caused by the deficiency of arylsulfatase B, received marketing approval in the U.S. in May 2005, in the EU in January 2006 and subsequently in other countries. Naglazyme net product revenues for the three and nine months ended September 30, 2014 totaled \$67.5 million and \$246.0 million, respectively, compared to \$63.2 million and \$202.5 million for the three and nine months ended September 30, 2013, respectively.

Kuvan was granted marketing approval for the treatment of phenylketonuria (PKU) in the U.S. in December 2007 and in the EU in December 2008. Kuvan net product revenues for the three and nine months ended September 30, 2014 totaled \$53.4 million and \$145.6 million, respectively, compared to \$43.6 million and \$122.1 million for the three and nine months ended September 30, 2013, respectively.

Aldurazyme, which was developed in collaboration with Genzyme Corporation (Genzyme), was approved in 2003 for marketing in the U.S. and the EU and subsequently in other countries for patients with mucopolysaccharidosis I (MPS I). Aldurazyme net product revenues for the three and nine months ended September 30, 2014 totaled \$22.6 million and \$64.7 million, respectively, compared to \$23.4 million and \$57.7 million for the three and nine months ended September 30, 2013, respectively.

In December 2009, the European Medicines Agency (EMA) granted marketing approval for Firdapse, a proprietary form of 3-4-diaminopyridine (amifampridine phosphate), for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS). We launched this product on a country-by-country basis in the EU beginning in April 2010. Firdapse net product revenues for the three and nine months ended September 30, 2014 totaled \$4.7 million and \$14.0 million, respectively, compared to \$4.1 million and \$11.8 million for the three and nine months ended September 30, 2013, respectively.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including:

PEG PAL, an enzyme substitution therapy for the treatment of PKU;

BMN 701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder;

Talazoparib (BMN 673), an orally available poly-ADP ribose polymerase inhibitor for the treatment of patients with certain cancers;

BMN 111, a peptide therapeutic for the treatment of achondroplasia, the leading cause of dwarfism; and

BMN 190 for the treatment of late infantile neuronal ceroid lipofuscinosis (CLN2), a lysosomal storage disorder primarily affecting the brain.

We are conducting or planning to conduct preclinical development of several other product candidates for genetic and other metabolic diseases and recently announced the selection of two new drug development candidates, BMN 270 and BMN 250. BMN 270 is a Factor VIII gene therapy drug development candidate, an AAV VIII vector, for the treatment of hemophilia A. BMN 250 is a novel fusion of alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome, or Mucopolysaccharidosis type IIIB (MPS IIIB).

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### **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.

Research and development 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.

Selling, general and administrative 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.

Intangible asset amortization and contingent consideration includes amortization expense related to our finite-lived intangible assets associated with marketing rights in the EU for Firdapse, amortization of intangible assets related to SRCC in-place and above market leases, impairment losses (if any) on intangible assets and changes in the fair value of contingent acquisition consideration payable. Changes in fair value can result from changes in estimated probability adjustments, changes in estimated timing of when a milestone may be achieved, changes in assumed discount periods and rates.

Our cash, cash equivalents, short-term investments and long-term investments totaled \$1,114.7 million as of September 30, 2014, compared to \$1,052.4 million as of December 31, 2013. We have historically financed our operations primarily through our cash flows from operating activities, the issuance of common stock and convertible debt and by relying on equipment and other commercial financing. 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. 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 Securities and Exchange Commission (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 historical 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.



**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)**

There have been no significant changes to our critical accounting policies and estimates during nine months ended September 30, 2014, 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, 2013, which was filed with the SEC on February 26, 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.

**Results of Operations*****Net Income (Loss)***

Our net income for the three months ended September 30, 2014 was \$7.4 million, compared to a net loss of \$53.0 million for the three months ended September 30, 2013. Our net loss for the nine months ended September 30, 2014 was \$64.2 million, compared to a net loss of \$114.4 million for the nine months ended September 30, 2013. The decrease in net loss was primarily a result of the following (in millions):

|                                                                                     | <b>Three<br/>Months</b> | <b>Nine<br/>Months</b> |
|-------------------------------------------------------------------------------------|-------------------------|------------------------|
| Net loss for the period ended September 30, 2013                                    | \$ (53.0)               | \$ (114.4)             |
| Gain on sale of intangible asset                                                    | 67.5                    | 67.5                   |
| Increased gross profit from product sales                                           | 37.3                    | 103.8                  |
| Increased research and development expense                                          | (37.6)                  | (62.1)                 |
| Increased selling, general and administrative expense                               | (12.8)                  | (39.0)                 |
| Increased interest expense                                                          | (8.6)                   | (24.6)                 |
| Increased benefit (provision) for income taxes                                      | 4.9                     | (7.8)                  |
| Decreased (increased) intangible asset amortization and<br>contingent consideration | 6.7                     | (2.6)                  |
| Decreased debt conversion expense                                                   | 1.7                     | 11.5                   |
| Other individually insignificant fluctuations                                       | 1.3                     | 3.5                    |
| <b>Net income (loss) for the period ended September 30, 2014</b>                    | <b>\$ 7.4</b>           | <b>\$ (64.2)</b>       |

In July 2014, we sold the Rare Pediatric Disease Priority Review Voucher (PRV) we received in connection with the approval of VIMIZIM. In exchange for the voucher we received \$67.5 million from Regeneron Ireland (Regeneron), an indirect, wholly-owned subsidiary of Regeneron Pharmaceuticals, Inc. The proceeds from the sale of PRV were recognized as a gain on the sale of intangible asset. The increase in gross profit from product sales during the three and nine months ended September 30, 2014 as compared to the three and nine months ended September 30, 2013 was primarily a result of the commercial launch of VIMIZIM, additional Kuvan patients initiating therapy in the U.S., and

additional Naglazyme patients initiating therapy globally. The increase in research and development expense was primarily attributed to the clinical trials of our late-stage development programs, licensing fees paid to a third-party to secure licenses related to the development of BMN 673 and increased research on earlier stage development programs. The increase in selling, general and administrative expense was primarily due to increased sales and marketing expenses related to our commercial products and increased expenses related to the commercial launch of VIMIZIM.

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

**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)*****Net Product Revenues, Cost of Sales and Gross Profit***

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

|                            | <b>Three Months Ended<br/>September 30,</b> |             |               | <b>Nine Months Ended<br/>September 30,</b> |             |               |
|----------------------------|---------------------------------------------|-------------|---------------|--------------------------------------------|-------------|---------------|
|                            | <b>2014</b>                                 | <b>2013</b> | <b>Change</b> | <b>2014</b>                                | <b>2013</b> | <b>Change</b> |
| VIMIZIM                    | \$ 25.2                                     | \$ 0        | \$ 25.2       | \$ 40.4                                    | \$ 0        | \$ 40.4       |
| Naglazyme                  | 67.5                                        | 63.2        | 4.3           | 246.0                                      | 202.5       | 43.5          |
| Kuvan                      | 53.4                                        | 43.6        | 9.8           | 145.6                                      | 122.1       | 23.5          |
| Aldurazyme                 | 22.6                                        | 23.4        | (0.8)         | 64.7                                       | 57.7        | 7.0           |
| Firdapse                   | 4.7                                         | 4.1         | 0.6           | 14.0                                       | 11.8        | 2.2           |
| Total net product revenues | \$ 173.4                                    | \$ 134.3    | \$ 39.1       | \$ 510.7                                   | \$ 394.1    | \$ 116.6      |

Gross profit by product was as follows (in millions):

|                    | <b>Three Months Ended<br/>September 30,</b> |             |               | <b>Nine Months Ended<br/>September 30,</b> |             |               |
|--------------------|---------------------------------------------|-------------|---------------|--------------------------------------------|-------------|---------------|
|                    | <b>2014</b>                                 | <b>2013</b> | <b>Change</b> | <b>2014</b>                                | <b>2013</b> | <b>Change</b> |
| VIMIZIM            | \$ 22.0                                     | \$ 0        | \$ 22.0       | \$ 35.7                                    | \$ 0        | \$ 35.7       |
| Naglazyme          | 57.5                                        | 54.1        | 3.4           | 211.4                                      | 173.6       | 37.8          |
| Kuvan              | 44.8                                        | 36.1        | 8.7           | 122.9                                      | 102.5       | 20.4          |
| Aldurazyme         | 15.6                                        | 12.9        | 2.7           | 46.2                                       | 37.7        | 8.5           |
| Firdapse           | 3.6                                         | 3.1         | 0.5           | 10.6                                       | 9.2         | 1.4           |
| Total gross profit | \$ 143.5                                    | \$ 106.2    | \$ 37.3       | \$ 426.8                                   | \$ 323.0    | \$ 103.8      |

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

|                                        | <b>Three Months Ended<br/>September 30,</b> |             |               | <b>Nine Months Ended<br/>September 30,</b> |             |               |
|----------------------------------------|---------------------------------------------|-------------|---------------|--------------------------------------------|-------------|---------------|
|                                        | <b>2014</b>                                 | <b>2013</b> | <b>Change</b> | <b>2014</b>                                | <b>2013</b> | <b>Change</b> |
| Aldurazyme revenue reported by Genzyme | \$ 54.3                                     | \$ 50.8     | \$ 3.5        | \$ 172.5                                   | \$ 152.9    | \$ 19.6       |

**Three Months Ended  
September 30,**

**Nine Months Ended  
September 30,**

|                                                                         | <b>2014</b>    | <b>2013</b>    | <b>Change</b>   | <b>2014</b>    | <b>2013</b>    | <b>Change</b> |
|-------------------------------------------------------------------------|----------------|----------------|-----------------|----------------|----------------|---------------|
| Royalties earned from Genzyme                                           | \$ 22.9        | \$ 20.5        | \$ 2.4          | \$ 69.1        | \$ 61.0        | \$ 8.1        |
| Incremental (previously recognized) Aldurazyme product transfer revenue | (0.3)          | 2.9            | (3.2)           | (4.4)          | (3.3)          | (1.1)         |
| <b>Total Aldurazyme net product revenues</b>                            | <b>\$ 22.6</b> | <b>\$ 23.4</b> | <b>\$ (0.8)</b> | <b>\$ 64.7</b> | <b>\$ 57.7</b> | <b>\$ 7.0</b> |

Net product revenues for Naglazyme for the three and nine months ended September 30, 2014 totaled \$67.5 million and \$246.0 million, respectively, of which \$58.1 million and \$216.8 million, respectively, was earned from customers based outside the U.S., compared to \$63.2 million and \$202.5 million for the three and nine months ended September 30, 2013, respectively, of which \$54.4 million and \$174.7 million, respectively, was earned from customers based outside the U.S. The increase in Naglazyme net product revenues for the three and nine months ended September 30, 2014 was attributed to new patients initiating therapy. The impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than the U.S. dollar was positive by \$0.2 million and \$1.3 million for the three and nine months ended September 30, 2014. Naglazyme gross margins were 85% for each of the three months ended September 30, 2014 and 2013 and 86% for each of the nine months ended September 30, 2014 and 2013. Naglazyme gross margins for the three and nine months ended September 30, 2014 were consistent with expectations and are not expected to fluctuate significantly in the future.

**Table of Contents****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, 2014 totaled \$53.4 million and \$145.6 million, respectively, compared to \$43.6 million and \$122.1 million for the three and nine months ended September 30, 2013, respectively. The increase in Kuvan net product revenues was attributed to new patients initiating therapy. Kuvan gross margins were 84% for each of the three and nine months ended September 30, 2014, compared to 83% and 84% for the three and nine months ended September 30, 2013, respectively. Cost of goods sold for each of the three and nine months ended September 30, 2014 and 2013 reflect royalties paid to third-parties of approximately 10%. Kuvan gross margins for the three and nine months ended September 30, 2014 were consistent with expectations and are not expected to fluctuate significantly in the future. The royalties earned from Merck Serono's net sales of Kuvan for the three and nine months ended September 30, 2014 were \$0.5 million and \$1.7 million, respectively, compared to \$0.6 million and \$1.6 million for the three and nine months ended September 30, 2013, respectively.

The Hatch-Waxman Act permits the FDA to approve abbreviated new drug applications (ANDA), for the generic versions of branded drugs. We have received a paragraph iv notice letter (the Notice Letter), dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (DRL), notifying us that DRL has 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 FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). We have eight patents listed in the FDA Orange Book with expiration dates between 2024 and 2026. We are evaluating DRL's allegations and will take appropriate action, which may include suing DRL for patent infringement within 45 days of receipt of the Notice Letter. By statute, if we initiate a patent infringement lawsuit against the party submitting the letter within 45 days of receiving the notice letter, then the FDA would automatically be precluded from approving the ANDA for 30 months, or until a district court decision finding the patents invalid or not infringed, whichever occurs earlier. Once the lawsuit is filed, the 30 month stay period will begin as of the date we are notified of the filing. If the generic competitor were to enter the market following the expiration of the orphan exclusivity, which expires at the end of November 2014, it could have an adverse effect on our Kuvan net product revenues.

Aldurazyme gross margins were 69% and 71% for the three and nine months ended September 30, 2014, respectively, compared to 55% and 65% for the three and nine months ended September 30, 2013, 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.

Net product revenue for Firdapse for the three and nine months ended September 30, 2014 totaled \$4.7 million and \$14.0 million, respectively, compared to \$4.1 million and \$11.8 million for the three and nine months ended September 30, 2013, respectively. Firdapse gross margins for the three and nine months ended September 30, 2014 were 77% and 76%, respectively, compared to 77% and 78% for the three and nine months ended September 30, 2013, respectively. Cost of goods sold for each of the three and nine months ended September 30, 2014 and 2013 reflect royalties paid to third-parties of approximately 8%. Firdapse gross margins for the three and nine months ended September 30, 2014 decreased due to increased manufacturing costs. Firdapse gross margins for the three and nine months ended September 30, 2014 were consistent with expectations and are not expected to fluctuate significantly in the future.

The FDA and the EMA granted marketing approval for VIMIZIM in February 2014 and April 2014, respectively, and we began marketing the product immediately following approval in each of these markets. Net product revenues for VIMIZIM for the three and nine months ended September 30, 2014 totaled \$25.2 million and \$40.4 million,

respectively, of which \$11.3 million and \$16.5 million, respectively, was earned from customers based outside the U.S. VIMIZIM gross margins were 87% and 88% for the three and nine months ended September 30, 2014, respectively. In future periods, we expect VIMIZIM gross margins to decline and approximate Naglazyme gross margins as we deplete previously expensed product.

**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)**

Total cost of sales for the three and nine months ended September 30, 2014 were \$29.9 million and \$83.9 million, respectively, compared to \$28.1 million and \$71.1 million for the three and nine months ended September 30, 2013, respectively. The increase in cost of sales was primarily attributed to the increase in product sales.

***Research and Development***

We manage our research and development 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.

Research and development expense increased to \$125.7 million for the three months ended September 30, 2014, from \$88.1 million for the three months ended September 30, 2013. Research and development expense increased to \$319.6 million for the nine months ended September 30, 2014, from \$257.5 million for the nine months ended September 30, 2013. The increase in research and development expense was primarily a result of the following (in millions):

|                                                                                 | <b>Three<br/>Months</b> | <b>Nine<br/>Months</b> |
|---------------------------------------------------------------------------------|-------------------------|------------------------|
| Research and development expense for the period ended September 30, 2013        | \$ 88.1                 | \$ 257.5               |
| Gain on early lease termination                                                 | 0                       | (6.6)                  |
| Increased BMN 673 development expenses                                          | 17.5                    | 24.0                   |
| Increased BMN 190 development expenses                                          | 8.3                     | 15.9                   |
| Increased development expenses on early development stage programs              | 11.0                    | 19.8                   |
| Increased PEG PAL development expenses                                          | 1.1                     | 9.8                    |
| Increased BMN 111 development expenses                                          | 2.7                     | 4.0                    |
| Decreased VIMIZIM development expenses                                          | (4.6)                   | (11.9)                 |
| Increased (decreased) BMN 701 development expenses                              | 1.0                     | (1.1)                  |
| Decreased development expenses related to mature commercial products            | (2.4)                   | (5.2)                  |
| Increased non-allocated research and development expenses and other net changes | 3.0                     | 13.4                   |
| Research and development expense for the period ended September 30, 2014        | \$ 125.7                | \$ 319.6               |

The increase in BMN 673 development expense was attributed to increased clinical trial activities related to this product candidate and upfront licensing fees of \$11.5 million paid to a third-party to secure licenses related to the development of BMN 673. The increase in PEG PAL development expense was attributed to increased clinical trial activities related to this product candidate. 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 and development costs related to the programs acquired from Zacharon. The increases in BMN 190 and BMN 111 development expense were attributed to increased pre-clinical activities related to these product candidates. The increase in BMN 701 development expense during the three months ended September 30, 2014 was attributed to increased clinical trial

activities related to this product. The decrease in BMN 701 development expense for the nine months ended September 30, 2014 was attributed to a decline in clinical manufacturing costs, offset by an increase in clinical trial expense. The increase in non-allocated research and development expense is primarily attributed to an increase in research and development personnel costs and facility costs that are not allocated to specific programs. The gain on early lease termination in the nine months ended September 30, 2014, was primarily due to the early termination of our lease and the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of SRCC where our corporate headquarters are located.

During the remainder of 2014, we expect our research and development spending to increase over 2013 levels due to our PEG PAL, BMN 673, BMN 701, BMN 111 and BMN 190 programs progressing, including a few of those programs progressing to more advanced phases of clinical studies. Phase 3 clinical trials for PEG PAL and BMN 673 were initiated in the second and fourth quarters of 2013, respectively, and we initiated a Phase 3 trial of BMN 701 in the second quarter of 2014. We also expect increased spending on pre-clinical and clinical activities for



**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)**

our early development stage programs including BMN 270, programs acquired from Zacharon and BMN 250. Additionally, we expect to continue incurring significant research and development 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 research and development expenses.

***Selling, General and Administrative***

Selling, general and administrative expense increased to \$74.6 million for the three months ended September 30, 2014, from \$61.8 million for the three months ended September 30, 2013. Selling, general and administrative expense increased to \$202.5 million for the nine months ended September 30, 2014, from \$163.5 million for the nine months ended September 30, 2013. The increase in selling, general and administrative expenses was primarily a result of the following (in millions):

|                                                                                     | <b>Three<br/>Months</b> | <b>Nine<br/>Months</b> |
|-------------------------------------------------------------------------------------|-------------------------|------------------------|
| Selling, general and administrative expense for the period ended September 30, 2013 | \$ 61.8                 | \$ 163.5               |
| Gain on early lease termination                                                     | 0                       | (2.7)                  |
| Increased VIMIZIM commercial launch expenses                                        | 5.7                     | 18.3                   |
| Increased stock-based compensation                                                  | 2.9                     | 8.2                    |
| Decreased sales and marketing expenses related to mature commercial products        | (1.4)                   | (1.9)                  |
| Net increase in corporate support and other administrative expenses                 | 5.6                     | 17.1                   |
| Selling, general and administrative expense for the period ended September 30, 2014 | \$ 74.6                 | \$ 202.5               |

The gain on early lease termination in the nine months ended September 30, 2014, resulted from the early termination of our lease and the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of the SRCC where our corporate headquarters are located. The increase in stock-based compensation is attributed to an increase in the number of options outstanding due to an increased number of employees and an increase in the weighted-average fair value of the equity awards granted during 2014. We continue to incur sales and marketing expense for Naglazyme and Kuvan as a result of continued expansion of our international and U.S. activities, respectively. The increase in corporate support and other administrative expenses is primarily attributed to employee related expenses due to the increased number of employees. We expect selling, general and administrative expenses 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 the European marketing rights for Firdapse and intangible assets related to SRCC in-place and above market leases. 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):

**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)**

|                                                                           | <b>Three Months Ended<br/>September 30,</b> |               |                 | <b>Nine Months Ended<br/>September 30,</b> |                |               |
|---------------------------------------------------------------------------|---------------------------------------------|---------------|-----------------|--------------------------------------------|----------------|---------------|
|                                                                           | <b>2014</b>                                 | <b>2013</b>   | <b>Change</b>   | <b>2014</b>                                | <b>2013</b>    | <b>Change</b> |
| Changes in the fair value of contingent acquisition consideration payable | \$ 1.8                                      | \$ 8.8        | \$ (7.0)        | \$ 12.6                                    | \$ 9.9         | \$ 2.7        |
| Impairment loss on intangible assets                                      | 0                                           | 0             | 0               | 0                                          | 0.9            | (0.9)         |
| Amortization of Firdapse European marketing rights                        | 0.8                                         | 0.8           | 0               | 2.4                                        | 2.4            | 0             |
| Amortization of SRCC in-place and above market leases                     | 0.3                                         | 0             | 0.3             | 0.8                                        | 0              | 0.8           |
| <b>Total intangible asset amortization and contingent consideration</b>   | <b>\$ 2.9</b>                               | <b>\$ 9.6</b> | <b>\$ (6.7)</b> | <b>\$ 15.8</b>                             | <b>\$ 13.2</b> | <b>\$ 2.6</b> |

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 three and nine months ended September 30, 2014, the majority of the changes related to the development progress of BMN 701.

In the second quarter of 2013, we recorded an impairment charge of \$0.9 million related to in-process research and development (IPR&D) assets related to acquired pre-clinical compounds based on the status of current development efforts and the related discounted cash flows that no longer supported the carrying-value of the IPR&D assets.

***Equity in the Loss of BioMarin/Genzyme LLC***

Equity in the loss of BioMarin/Genzyme LLC (the LLC) includes our 50% share of the LLC's loss for the period. The LLC's operations consist primarily of certain research and development activities and the intellectual property that are managed by the LLC, with costs shared equally by BioMarin and Genzyme.

Equity in the loss of the LLC totaled \$0.2 million and \$1.1 million for the three and nine months ended September 30, 2014, respectively, compared to \$0.1 million and \$0.7 million for the three and nine months ended September 30, 2013, respectively.

***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 \$1.4 million and \$4.3 million for the three and nine months ended September 30, 2014, respectively, compared to \$0.6 million and \$1.9 million for the three and nine months ended September 30, 2013, respectively. The increase in interest income during the three and nine months ended September 30, 2014, as compared to the three and nine months ended September 30, 2013 was primarily due to higher cash and investment balances. We do not expect future interest income to fluctuate significantly over the next twelve months.



**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)*****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<br>September 30, |        |        | Nine Months Ended<br>September 30, |        |         |
|--------------------------------------------|-------------------------------------|--------|--------|------------------------------------|--------|---------|
|                                            | 2014                                | 2013   | Change | 2014                               | 2013   | Change  |
| Coupon interest                            | \$ 2.3                              | \$ 0.5 | \$ 1.8 | \$ 7.1                             | \$ 2.5 | \$ 4.6  |
| Amortization of issuance costs             | 0.8                                 | 0      | 0.8    | 2.5                                | 0.4    | 2.1     |
| Accretion of discount on convertible notes | 6.0                                 | 0      | 6.0    | 17.8                               | 0      | 17.8    |
| Total interest expense                     | \$ 9.1                              | \$ 0.5 | \$ 8.6 | \$ 27.4                            | \$ 2.9 | \$ 24.5 |

The increased interest expense in the three and nine months ended September 30, 2014, compared to the three and nine months ended September 30, 2013 was attributed to our October 2013 debt offering. We expect future interest expense to increase due to the October 2013 debt offering and the accretion of the related debt discount. See Note 5 to the Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2013, for additional information regarding our Convertible Debt.

During the nine months ended September 30, 2014, we recognized Debt Conversion Expense of \$0.7 million in connection with the early conversion of \$3.5 million and \$13.0 million in aggregate principal amount of our senior subordinated convertible notes due in 2017 (the 2017 Notes) in April 2014 and June 2014, respectively. During the three and nine months ended September 30, 2013, we recognized debt conversion expense of \$1.7 million and \$12.2 million, respectively in connection with the early conversion \$215.0 million and \$31.5 million in aggregate principal amount of the 2017 Notes, in March 2013 and August 2013, respectively.

***Provision for (Benefit from) Income Taxes***

During the three and nine months ended September 30, 2014 we recognized income tax benefit of \$4.2 million and income tax expense of \$5.0 million, respectively, compared to income tax expense of \$0.7 million and income tax benefit of \$2.8 million during the three and nine months ended September 30, 2013, respectively. Income tax expense/benefit for the three and nine months ended September 30, 2014 and 2013 consisted of state, federal and foreign current tax expense, which were offset by deferred tax benefits from federal orphan drug credits and California research and development (R&D) credits. The income tax expense/benefit for the three and nine months ended September 30, 2013 were also reduced by the federal R&D credit, which have not been reinstated for 2014 as of September 30, 2014. The income tax expense/benefit for the three and nine months ended September 30, 2014 and 2013 were further reduced by the benefit related to stock option exercises during these periods. Additionally, the American Taxpayer Relief Act of 2012 (the Relief Act), was enacted on January 2, 2013. The Relief Act reinstated the federal R&D credit retroactively to January 1, 2012. In accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 740, Income Taxes, we accounted for the effects of change in the tax law in the period that included the enactment date of the change, resulting in the recognition of a deferred tax benefit of \$1.2

million related to R&D expenses incurred during 2012 as a discrete item during the nine months ended September 30, 2013. The discrete benefits for the nine months ended September 30, 2014 were partially offset by a \$0.6 million increase in the valuation allowance related to California net operating losses that we believe are likely to expire unutilized. See Note 20 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2013 for additional discussion of the components of our provision for (benefit from) income taxes.

### **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

**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)**

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 October 2013 debt offering and our March 2014 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, 2014, \$162.6 million of our \$1,114.7 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 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 adjust our business processes, as appropriate, to mitigate these risks to our business.

Our financial condition as of September 30, 2014 and December 31, 2013 was as follows (in millions):

|                                        | <b>September 30,<br/>2014</b> | <b>December 31,<br/>2013</b> | <b>Change</b> |
|----------------------------------------|-------------------------------|------------------------------|---------------|
| Cash and cash equivalents              | \$ 398.0                      | \$ 568.8                     | \$ (170.8)    |
| Short-term investments                 | 268.4                         | 215.9                        | 52.5          |
| Long-term investments                  | 448.3                         | 267.7                        | 180.6         |
| Cash, cash equivalents and investments | \$ 1,114.7                    | \$ 1,052.4                   | \$ 62.3       |
| Current assets                         | \$ 1,077.8                    | \$ 1,137.4                   | \$ (59.6)     |
| Current liabilities                    | 206.6                         | 183.3                        | 23.3          |
| Working capital                        | \$ 871.2                      | \$ 954.1                     | \$ (82.9)     |

|                                   |          |          |        |
|-----------------------------------|----------|----------|--------|
| Convertible debt, net of discount | \$ 656.9 | \$ 655.6 | \$ 1.3 |
|-----------------------------------|----------|----------|--------|

Our cash flows for each of the nine months ended September 30, 2014 and 2013 are summarized as follows (in millions):

|                                                          | Nine Months Ended<br>September 30, |          |          |
|----------------------------------------------------------|------------------------------------|----------|----------|
|                                                          | 2014                               | 2013     | Change   |
| Cash and cash equivalents at the beginning of the period | \$ 568.8                           | \$ 180.5 | \$ 388.3 |
| Net cash used in operating activities                    | (46.8)                             | (48.3)   | 1.5      |
| Net cash (used in) provided by investing activities      | (266.7)                            | 7.6      | (234.3)  |
| Net cash provided by financing activities                | 142.7                              | 41.8     | 100.9    |
| Cash and cash equivalents at the end of the period       | \$ 398.0                           | \$ 181.6 | \$ 216.4 |
| Short-term and long-term investments                     | 716.7                              | 325.6    | 391.1    |
| Cash, cash equivalents and investments                   | \$ 1,114.7                         | \$ 507.2 | \$ 607.5 |

### *Cash, Cash Equivalents and Investments*

The increase in cash, cash equivalents and investments at September 30, 2014 from December 31, 2013 was primarily attributed to the net proceeds of \$117.5 million from our March 2014 public offering of common stock and employee stock option exercises and ESPP contributions, offset by increases in cash used in operating activities and purchases of property, plant and equipment.



**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)*****Working Capital***

Working capital decreased by \$82.9 million, from \$954.1 million at December 31, 2013 to \$871.2 million at September 30, 2014. The decrease in working capital was attributed to the following (in millions):

|                                                             |              |
|-------------------------------------------------------------|--------------|
| Working capital at December 31, 2013                        | \$ 954.1     |
| Decreased cash, cash equivalents and short-term investments | (118.3)      |
| Increased accounts payable and accrued liabilities          | (23.3)       |
| Net increase in other current operating assets              | 58.7         |
| <br>Working capital at September 30, 2014                   | <br>\$ 871.2 |

The decrease in cash, cash equivalents and short-term investments was primarily attributed to the increase in long-term investments offset by proceeds of \$37.6 million from employee stock option exercises and ESPP contributions.

The net increase in other current operating assets is attributed to a \$41.8 million increase in inventory, a \$3.6 million increase in accounts receivable, a \$14.8 increase in other current assets, offset by a decrease of \$1.4 million in current deferred tax assets. The increase in inventory was primarily attributed to buildup of inventories for all commercial products during the nine months ended September 30, 2014. The increase in accounts receivable is attributed to timing of net product revenues and cash receipts from customers.

Our product sales to government-owned or government-funded customers in certain countries, including Russia and the Southern European countries of Greece, Spain, Italy and Portugal, are subject to payment terms that are imposed by government authority. 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 the Southern European countries or Russia, 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, 2014, approximately 9% of our outstanding accounts receivable relate to such countries. See Note 17 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, 2014 was \$46.8 million, compared to cash used in operating activities of \$48.3 million for the nine months ended September 30, 2013. The decrease in cash used in operating activities was primarily comprised of a \$20.1 million increase in accounts payable and accrued liabilities, and a \$12.1 million increase in collection of accounts receivable, offset by a \$21.9 million increase in inventory purchases, a \$8.0 million increase in other current assets, a \$6.4 million increase in other assets and a \$5.5 million decrease in other long-term liabilities.

***Cash (Used in) Provided by Investing Activities***

Net cash used in investing activities during the nine months ended September 30, 2014 was \$266.1 million compared to net cash provided by investing activities of \$7.6 million during the nine months ended September 30, 2013. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures, such as manufacturing equipment and facility improvements. The increase in net cash used in investing activities for the nine months ended September 30, 2014 was primarily comprised of a \$54.2 million

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

increase in capital expenditures and \$294.9 million in net purchases of investments, offset by a decrease of \$9.9 million in business acquisitions. During the remainder of 2014, 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 to accommodate anticipated headcount growth.

***Cash Provided by Financing Activities***

Net cash provided by financing activities for the nine months ended September 30, 2014 was \$142.7 million, compared to net cash provided by financing activities of \$41.8 million for the nine months ended September 30, 2013. Historically, our financing activities primarily included payments related to our contingent acquisition obligations, payments related to our convertible debt obligations and proceeds from employee stock purchases under the ESPP and employee stock option exercises. The increase in net cash provided by financing activities for the nine months ended September 30, 2014 was primarily attributed to of the net proceeds of \$117.5 million from our March 2014 equity offering and a \$11.5 million decrease in debt conversion expense, offset by decreased proceeds from employee stock option exercises and ESPP contributions of \$22.6 million and a \$4.7 million increase in payments of contingent acquisition consideration.

***Other Information***

In March 2014, we sold 1.5 million shares of our common stock at a price of \$78.45 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds of approximately \$117.5 million from this public offering.

On October 15, 2013, we completed an offering of \$750.0 million in aggregate principal amount of senior subordinated convertible notes consisting of \$375.0 million in aggregate principal amount of 0.75% senior subordinated convertible notes due 2018 (the 2018 Notes) and \$375.0 million in aggregate principal amount of the 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes and together with the 2018 Notes, the Notes). The net proceeds from the offering were \$696.4 million, after deducting commissions and offering expenses and the purchase of capped calls. The Notes were issued at face value and accrue interest at their stated annual rates which is payable semiannually in arrears on April 15 and October 15 of each year beginning on April 15, 2014. See Note 5 to the Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2013, for additional information regarding our Convertible Debt.

In April 2007, we sold approximately \$324.9 million of the 2017 Notes of which \$45.6 million remained outstanding at September 30, 2014. The debt was issued at face value and bears interest at the rate of 1.875% per annum, payable semi-annually in cash. During the nine months ended September 30, 2014, we entered into two separate agreements with one of the existing holders of the 2017 Notes pursuant to which such holder converted \$16.5 million in aggregate principal amount of the 2017 Notes into 809,351 shares of our common stock. In addition to issuing the requisite number of shares of common stock, we the also made varying cash payments to the holder totaling \$0.7 million in aggregate, of which \$0.7 million was recognized in total as Debt Conversion Expense on the Condensed Consolidated Statement of Comprehensive Income (Loss) for the nine months ended September 30, 2014.

During 2013, we entered into separate agreements with 18 of the existing holders of the 2017 Notes pursuant to which such holders converted \$262.8 million in aggregate principal of the 2017 Notes into 12.9 million shares of our

common stock. In addition to issuing the requisite number of shares of common stock pursuant to the 2017 Notes, we also made varying cash payments to each of the holders, totaling an aggregate of \$14.8 million, of which \$13.0 million was recognized as Debt Conversion Expense in our Consolidated Statement of Operations for the year ended December 31, 2013. The remaining 2017 Notes are convertible, at the option of the holder, at any time prior to maturity, into shares of our common stock at a conversion price of approximately \$20.36 per share, subject to adjustment in certain circumstances. The 2017 Notes do not contain a call provision and we are unable to unilaterally redeem the 2017 Notes prior to maturity in 2017. We also must repay the debt if there is a qualifying change in control or termination of trading of our common stock. If a change of control occurs, we will pay a make whole premium by increasing the conversion rate applicable to the 2017 Notes. See Note 5 to the Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2013, for additional information regarding our Convertible Debt.

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

In March 2006, we sold approximately \$172.5 million of our senior subordinated convertible notes due in March 2013 (the 2013 Notes), which fully matured on March 29, 2013. The debt was issued at face value and bore interest at the rate of 2.5% per annum, payable semi-annually in cash. The 2013 Notes did not contain a call provision and we were unable to unilaterally redeem the remaining debt prior to maturity in March 2013. Upon maturity of the 2013 Notes, we issued 1.4 million shares of our common stock pursuant to the terms of the 2013 Notes and paid a bond holder \$98,000 in cash for the par value at maturity. See Note 5 to the Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2013, for additional information regarding our Convertible Debt.

Our \$795.6 million (undiscounted) of total convertible debt as of September 30, 2014 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.

On January 4, 2013, we acquired Zacharon, which focused on developing small molecules targeting pathways of glycan and glycolipid metabolism, for a net cash upfront payment of \$9.7 million. In connection with the acquisition, we agreed to pay the Zacharon stockholders additional consideration in future periods of up to \$134.0 million (undiscounted) in milestone payments if certain clinical, development and sales milestones are met.

#### ***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.*

**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)**

Our investment in our product development programs and continued development of our existing commercial products has a major impact on our operating performance. Our research and development expenses in each of the three and nine months ended September 30, 2014 and 2013 and the period since inception of the major programs were as follows (in millions):

|                                                  | <b>Three Months Ended<br/>September 30,</b> |                | <b>Nine Months Ended<br/>September 30,</b> |                 | <b>Since Program<br/>Inception</b> |
|--------------------------------------------------|---------------------------------------------|----------------|--------------------------------------------|-----------------|------------------------------------|
|                                                  | <b>2014</b>                                 | <b>2013</b>    | <b>2014</b>                                | <b>2013</b>     |                                    |
| VIMIZIM                                          | \$ 15.3                                     | \$ 19.9        | \$ 49.8                                    | \$ 61.7         | \$ 343.6                           |
| BMN 673                                          | 26.0                                        | 8.5            | 42.7                                       | 18.7            | 99.3                               |
| BMN 701                                          | 10.9                                        | 9.9            | 35.2                                       | 36.3            | 132.4                              |
| BMN 111                                          | 5.9                                         | 3.2            | 14.9                                       | 10.9            | 61.8                               |
| BMN 190                                          | 11.5                                        | 3.2            | 25.6                                       | 9.7             | 57.1                               |
| PEG PAL                                          | 16.3                                        | 15.2           | 49.2                                       | 39.4            | 216.9                              |
| Mature approved products                         | 7.5                                         | 9.9            | 22.4                                       | 27.6            | 393.9                              |
| Not allocated to specific major current projects | 32.3                                        | 18.3           | 79.8                                       | 53.2            | Not meaningful                     |
| <b>Totals</b>                                    | <b>\$ 125.7</b>                             | <b>\$ 88.1</b> | <b>\$ 319.6</b>                            | <b>\$ 257.5</b> |                                    |

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;

manufacture, supply or distribution of VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse;

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;

government regulatory action affecting our product candidates or our competitors drug products in both the U.S. and in 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.



**Table of Contents****Management's Discussion and Analysis of Financial Condition and Results of Operations (Continued)*****Contractual and Commercial Obligations***

We have contractual and commercial obligations under our debt, operating leases and other obligations related to research and development activities, purchase commitments, licenses and sales royalties with annual minimums. Our contractual obligations for non-cancelable purchase commitments as of September 30, 2014 are presented in the table below (in millions).

|                                                   | <b>Payments Due within</b>      |                      |                      |                                  |                 |
|---------------------------------------------------|---------------------------------|----------------------|----------------------|----------------------------------|-----------------|
|                                                   | <b>Less<br/>Than 1<br/>Year</b> | <b>1-3<br/>Years</b> | <b>3-5<br/>Years</b> | <b>More<br/>Than 5<br/>Years</b> | <b>Total</b>    |
| 2017 Notes and related interest                   | \$ 0.4                          | \$ 1.8               | \$ 46.0              | \$ 0                             | \$ 48.2         |
| 2018 Notes and related interest                   | 1.4                             | 5.6                  | 380.6                | 0                                | 387.6           |
| 2020 Notes and related interest                   | 2.8                             | 11.2                 | 11.2                 | 386.3                            | 411.5           |
| Operating leases                                  | 1.4                             | 13.2                 | 3.6                  | 0.7                              | 18.9            |
| Research and development and purchase commitments | 33.2                            | 12.3                 | 0.5                  | 0                                | 46.0            |
| <b>Total</b>                                      | <b>\$ 39.2</b>                  | <b>\$ 44.1</b>       | <b>\$ 441.9</b>      | <b>\$ 387.0</b>                  | <b>\$ 912.2</b> |

We are also subject to contingent payments totaling approximately \$561.9 million as of September 30, 2014, which are due upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, \$57.9 million relates to programs that are no longer being developed.

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### **Item 3. Quantitative and Qualitative Disclosures About Market Risk**

Our market risks during the three months ended September 30, 2014 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, 2013, which was filed with the SEC on February 26, 2014.

### **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, regarding 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 that we file or submit under the Exchange Act is 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) 1992 Framework on internal control.

## **PART II. OTHER INFORMATION**

### **Item 1. Legal Proceedings.**

None.

### **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, 2013, which was filed with the SEC on February 26, 2014.



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**Risk 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 and Kuvan have received regulatory approval to be commercially marketed and sold in the U.S., EU and other countries. Firdapse has received regulatory approval to be commercially marketed only in the EU. VIMIZIM received regulatory approval in the U.S. on February 14, 2014, in the EU on April 28, 2014 and in Canada on July 7, 2014 but has not been approved in any other jurisdiction and may never receive additional regulatory approvals for any jurisdiction.

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, and record keeping. If we do not comply with the applicable regulations, the range of possible sanctions includes issuance of adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and 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

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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.

### **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 pharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication. 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, 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 (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 (collectively, 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

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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 abbreviated BLAs.

**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.

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 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 very 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 and 2013. 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.

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**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, we or our contract manufacturers, must obtain regulatory approval of our manufacturing facilities, processes and quality systems. 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 (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. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011 has not yet been approved by the FDA or the EMA. 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, 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 Good Manufacturing Practices (GMP) 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, 2014, we had cash, cash equivalents and short and long-term investments totaling \$1,114.7 million and long-term debt obligations of \$795.6 million (undiscounted). 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 the Notes but also the ongoing interest due on the Notes during their term. In March 2014, we sold 1.5 million shares of our common stock at a price of \$78.45 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds of \$117.5 million for this public offering; however 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);

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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 Therapeutics Inc., Huxley Pharmaceuticals, Inc., and Zacharon 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.

**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

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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 and Firdapse, 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 and Firdapse. We also rely on third-parties for portions of the manufacture of Naglazyme and Aldurazyme. 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.

**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;

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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, if approved outside of the U.S., 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 through special access or named

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patient programs, which do not require full product approval. We expect to also utilize these programs for VIMIZIM. 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.

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### **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 new 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 up to 2% per fiscal year, which went into effect on April 1, 2013. 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 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 payors. 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

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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 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. The Centers for Medicare and Medicaid Services has issued a final rule that requires manufacturers to begin collecting required information on August 1, 2013 with the first reports due March 31, 2014 (and by the 90th day of each calendar year thereafter) and publication of the reported data in a searchable form on a public website beginning September 30, 2014.

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 and Naglazyme 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:

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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.

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.

**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 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

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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 recently enacted America Invents Act, 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 the outcome is unpredictable. 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 BMN 673, BMN 701, BMN 111 and BMN 270, focus on therapeutic are 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.

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.

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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 BioMarin/Genzyme LLC (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.

**Based on our strategic alliance with Merck Serono, unless Merck Serono opts in to the PEG PAL program, we will not realize any cost sharing for the development expenses, development milestones, or royalties for ex-U.S.**

**sales.**

In May 2005, we entered into an agreement with Merck Serono for the further development and commercialization of Kuvan (and any other product containing 6R-BH<sub>4</sub>) and PEG PAL for PKU. Pursuant to that agreement, we received development milestones on Kuvan and receive royalties on sales by Merck Serono. Additionally, we may be entitled to development milestones and royalties related to PEG PAL. However, Merck

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Serono has opted out of the PEG PAL development program. Unless and until it elects to opt in, it is not obligated to pay any of the milestones related to the program or to reimburse us for any of the development costs. Additionally, even though Merck Serono has opted out of the PEG PAL development program, we do not have any right to commercialize PEG PAL outside of the U.S. and Japan or to grant anyone else such rights.

Merck Serono may elect to opt in at any time. If Merck Serono opts in to the PEG PAL development program before the unblinding of the first Phase 3 trial for PEG PAL, it must pay 75% of the Phase 3 costs incurred prior to the opt-in and the \$7,000,000 Phase 3 initiation milestone. If it opts in after unblinding of the first Phase 3 trial for PEG PAL, it must pay 100% of the Phase 3 costs incurred prior to the opt-in and the \$7,000,000 Phase 3 initiation milestone. Additionally, in all cases after it opts in to the PEG PAL development program, Merck Serono would be obligated to pay one half of future development costs under the agreement and any further milestones due under the agreement. If Merck Serono does not opt in, it will not have the right to use any of the clinical or other independently developed data.

We cannot determine when or if Merck Serono will opt in to the PEG PAL development program. If Merck Serono does not opt in, we will not receive any milestones under the agreement nor will there be any sales outside of the U.S. or Japan generating revenue from royalties or otherwise.

**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 product programs have been acquired through acquisitions, such as BMN 701 and BMN 673 and several of our 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 abbreviated new drug applications (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 Kuvan® (sapropterin hydrochloride) at any time after December 2011.

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BioMarin owns 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 that seeks FDA approval of its 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 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. Regardless of any litigation results, generic versions of Kuvan (sapropterin dihydrochloride) would be prohibited until the expiration of orphan drug exclusivity in June 2015, including pediatric exclusivity, at the earliest. 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 (sapropterin dihydrochloride) may be prohibited until October 2017.

We have received a paragraph iv notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (DRL), notifying us that DRL has filed of an ANDA seeking approval of a proposed generic version Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our Orange Book-listed patents (the Notice Letter). BioMarin is evaluating DRL's allegations and will take appropriate action, which may include suing DRL for patent infringement within 45 days of receipt of the Notice Letter.

The filing of DRL's ANDA application 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) are not upheld in litigation, or if DRL 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.

**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

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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 MPS I, MPS VI, PKU or 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 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 VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse, or our clinical trials for PEG PAL, BMN 701, BMN 673, BMN 111, BMN 190 or BMN 270 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

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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 three and nine months ended September 30, 2014 approximately 6% and 4%, respectively, of our net product revenues were from the countries of Italy, Spain, Portugal, Greece and Russia, respectively. Approximately 9% of our total accounts receivable as of September 30, 2014 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.

## **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:

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product sales and profitability of VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse;

manufacture, supply or distribution of VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse;

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;

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.

**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.

**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

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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., BioMarin GALNS Ltd. and Regeneron Ireland dated July 29, 2014.
- 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.

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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, 2014 and December 31, 2013, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three and nine months ended September 30, 2014 and 2013, (iii) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2014 and 2013, 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: October 28, 2014

By /S/ DANIEL SPIEGELMAN  
Daniel Spiegelman,

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

## **EXHIBIT INDEX**

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