

BIOMARIN PHARMACEUTICAL INC

Form 10-Q

November 03, 2016

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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, 2016

Or

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

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

Commission File Number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware 68-0397820  
(State or other jurisdiction of (I.R.S. Employer  
incorporation or organization) Identification No.)

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

(415) 506-6700

(Registrant's telephone number including area code)

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

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

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

Large accelerated filer

Accelerated filer

Non-accelerated filer (Do not check if a smaller reporting company) Smaller reporting company

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

Applicable only to corporate issuers:

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 171,987,437 shares of common stock, par value \$0.001, outstanding as of October 28, 2016.

## BIOMARIN PHARMACEUTICAL INC.

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BioMarin<sup>®</sup>, Vimizim<sup>®</sup>, Naglazyme<sup>®</sup>, Kuvan<sup>®</sup> and Firdapse<sup>®</sup> are our registered trademarks. Brineura<sup>™</sup> and Kyndris<sup>™</sup> are our trademarks. Aldurazyme<sup>®</sup> is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this report are the property of their respective owners.

## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED BALANCE SHEETS

September 30, 2016 and December 31, 2015

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

|   | September 30,<br>2016 | December 31,<br>2015 <sup>(1)</sup> |
|---|-----------------------|-------------------------------------|
| <b>ASSETS</b>   | (unaudited)           |                                     |
| Current assets:   |                       |                                     |
| Cash and cash equivalents   | \$ 707,349            | \$ 397,040                          |
| Short-term investments  | 327,499               | 195,579                             |
| Accounts receivable, net (allowance for doubtful accounts: \$66 and \$93,<br>at September 30, 2016 and December 31, 2015, respectively)   | 215,894               | 164,959                             |
| Inventory   | 347,420               | 271,683                             |
| Other current assets  | 68,409                | 60,378                              |
| Total current assets  | 1,666,571             | 1,089,639                           |
| Noncurrent assets:  |                       |                                     |
| Long-term investments   | 362,956               | 425,652                             |
| Property, plant and equipment, net  | 729,836               | 704,207                             |
| Intangible assets, net  | 561,387               | 683,996                             |
| Goodwill  | 197,039               | 197,039                             |
| Deferred tax assets   | 288,006               | 220,191                             |
| Other assets  | 36,443                | 408,644                             |
| Total assets  | \$ 3,842,238          | \$ 3,729,368                        |
| <b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>   |                       |                                     |
| Current liabilities:  |                       |                                     |
| Accounts payable and accrued liabilities  | \$ 306,129            | \$ 392,511                          |
| Short-term convertible debt, net  | 22,460                | —                                   |
| Short-term contingent acquisition consideration payable   | 48,746                | 52,946                              |
| Total current liabilities   | 377,335               | 445,457                             |
| Noncurrent liabilities:   |                       |                                     |
| Long-term convertible debt, net   | 653,178               | 662,286                             |
| Long-term contingent acquisition consideration payable  | 122,644               | 32,663                              |
| Deferred tax liabilities  | —                     | 143,527                             |
| Other long-term liabilities   | 43,273                | 44,588                              |
| Total liabilities   | 1,196,430             | 1,328,521                           |
| Stockholders' equity:   |                       |                                     |
| Common stock, \$0.001 par value: 250,000,000 shares authorized at<br>September 30, 2016 and December 31, 2015: 171,697,649 and 161,526,044 shares<br>issued and outstanding at September 30, 2016 and December 31, 2015, respectively | 173                   | 162                                 |
| Additional paid-in capital  | 4,231,514             | 3,414,837                           |
| Company common stock held by Nonqualified Deferred Compensation Plan (the<br>NQDC)  | (14,969 )             | (13,616 )                           |

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|  |              |              |
|--|--------------|--------------|
| Accumulated other comprehensive income     | 2,158        | 21,033       |
| Accumulated deficit                        | (1,573,068 ) | (1,021,569 ) |
| Total stockholders' equity                 | 2,645,808    | 2,400,847    |
| Total liabilities and stockholders' equity | \$ 3,842,238 | \$ 3,729,368 |

(1) December 31, 2015 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, 2015, filed with the Securities and Exchange Commission (the SEC) on February 29, 2016.

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

## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

Three and Nine Months Ended September 30, 2016 and 2015

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

(Unaudited)

|  | Three Months Ended<br>September 30, |                    | Nine Months Ended<br>September 30, |                     |
|--|-------------------------------------|--------------------|------------------------------------|---------------------|
|  | 2016                                | 2015               | 2016                               | 2015                |
| <b>REVENUES:</b>   |                                     |                    |                                    |                     |
| Net product revenues                                       | \$278,262                           | \$207,767          | \$812,195                          | \$658,102           |
| Collaborative agreement revenues                           | 1                                   | 131                | 234                                | 849                 |
| Royalty, license and other revenues                        | 1,633                               | 1,006              | 4,334                              | 3,008               |
| Total revenues   | 279,896                             | 208,904            | 816,763                            | 661,959             |
| <b>OPERATING EXPENSES:</b>                                 |                                     |                    |                                    |                     |
| Cost of sales  | 50,738                              | 34,904             | 145,473                            | 103,965             |
| Research and development                                   | 160,831                             | 158,713            | 486,663                            | 458,688             |
| Selling, general and administrative                        | 118,758                             | 94,044             | 333,635                            | 288,364             |
| Intangible asset amortization and contingent consideration | 9,654                               | 3,116              | (34,318 )                          | 22,963              |
| Impairment of intangible assets                            | —                                   | —                  | 599,118                            | —                   |
| Total operating expenses                                   | 339,981                             | 290,777            | 1,530,571                          | 873,980             |
| <b>LOSS FROM OPERATIONS</b>                                | <b>(60,085 )</b>                    | <b>(81,873 )</b>   | <b>(713,808 )</b>                  | <b>(212,021 )</b>   |
| Equity in the loss of BioMarin/Genzyme LLC                 | (104 )                              | (186 )             | (374 )                             | (539 )              |
| Interest income  | 1,633                               | 1,344              | 4,561                              | 3,050               |
| Interest expense   | (9,980 )                            | (9,447 )           | (29,767 )                          | (28,911 )           |
| Debt conversion expense                                    | —                                   | —                  | —                                  | (163 )              |
| Other income (expense)                                     | 1,723                               | (281 )             | 504                                | (9,105 )            |
| <b>LOSS BEFORE INCOME TAXES</b>                            | <b>(66,813 )</b>                    | <b>(90,443 )</b>   | <b>(738,884 )</b>                  | <b>(247,689 )</b>   |
| Provision for (benefit from) income taxes                  | (24,016 )                           | 483                | (187,385 )                         | (7,273 )            |
| <b>NET LOSS</b>  | <b>\$(42,797 )</b>                  | <b>\$(90,926 )</b> | <b>\$(551,499 )</b>                | <b>\$(240,416 )</b> |
| <b>NET LOSS PER SHARE, BASIC</b>                           | <b>\$(0.26 )</b>                    | <b>\$(0.57 )</b>   | <b>\$(3.36 )</b>                   | <b>\$(1.51 )</b>    |
| <b>NET LOSS PER SHARE, DILUTED</b>                         | <b>\$(0.26 )</b>                    | <b>\$(0.60 )</b>   | <b>\$(3.37 )</b>                   | <b>\$(1.51 )</b>    |
| Weighted average common shares outstanding, basic          | 167,714                             | 160,886            | 163,963                            | 159,647             |
| Weighted average common shares outstanding, diluted        | 167,714                             | 161,134            | 164,216                            | 159,647             |
| <b>COMPREHENSIVE LOSS</b>                                  |                                     |                    |                                    |                     |
|  | <b>\$(45,167 )</b>                  | <b>\$(98,203 )</b> | <b>\$(570,374 )</b>                | <b>\$(242,698 )</b> |

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



## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED STATEMENT OF STOCKHOLDERS' EQUITY

Nine Months Ended September 30, 2016

(In thousands of U.S. dollars)

(Unaudited)

|  | Common stock |        | Additional<br>Paid-in<br>Capital | Company<br>Common<br>Stock<br>Held by<br>the<br>NQDC | Accumulated<br>Other<br>Comprehensive<br>Income<br>(Loss) | Accumulated<br>Deficit | Total<br>Stockholders'<br>Equity |
|--|--------------|--------|----------------------------------|--|---|------------------------|----------------------------------|
|  | Shares       | Amount |                                  |  |   |                        |                                  |
| Balance at December 31, 2015                         | 161,526      | \$ 162 | \$3,414,837                      | \$(13,616)   | \$ 21,033   | \$(1,021,569)          | \$ 2,400,847                     |
| Net loss   |              |        |                                  |  |   | (551,499)              | (551,499)                        |
| Other comprehensive loss                             |              |        |                                  |  | (18,875)  |                        | (18,875)                         |
| Issuance of common stock, net of offering costs      | 7,500        | 8      | 712,930                          |  |   |                        | 712,938                          |
| Issuance of common stock under the 2006              |              |        |                                  |  |   |                        |                                  |
| Employee Stock Purchase Plan (the ESPP)              | 110          |        | 6,048                            |  |   |                        | 6,048                            |
| Exercise of common stock options                     | 1,345        | 1      | 43,449                           |  |   |                        | 43,450                           |
| Excess tax benefit from stock option exercises       |              |        | 563                              |  |   |                        | 563                              |
| Restricted stock units vested during the period, net | 779          | 1      | (55,242)                         |  |   |                        | (55,241)                         |
| Conversion of convertible notes, net                 | 438          | 1      | 8,923                            |  |   |                        | 8,924                            |
| Common stock held by the NQDC                        |              |        |                                  | (1,353)  |   |                        | (1,353)                          |
| Stock-based compensation                             |              |        | 100,006                          |  |   |                        | 100,006                          |
| Balance at September 30, 2016                        | 171,698      | \$ 173 | \$4,231,514                      | \$(14,969)   | \$ 2,158  | \$(1,573,068)          | \$ 2,645,808                     |

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





## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Nine Months Ended September 30, 2016 and 2015

(In thousands of U.S. dollars)

(Unaudited)

|  | 2016        | 2015         |
|--|-------------|--------------|
| <b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>                                       |             |              |
| Net loss   | \$(551,499) | \$(240,416 ) |
| Adjustments to reconcile net loss to net cash used in operating activities:        |             |              |
| Depreciation and amortization  | 76,805      | 34,301       |
| Non-cash interest expense  | 22,276      | 21,243       |
| Accretion of discount on investments   | 681         | 1,616        |
| Stock-based compensation   | 97,220      | 80,959       |
| (Gain) loss on sale of equity investment   | 2,020       | (3,022 )     |
| Impairment of assets   | 599,118     | 12,802       |
| Deferred income taxes  | (206,691)   | 14,629       |
| Excess tax benefit from stock option exercises                                     | (563 )      | (463 )       |
| Unrealized foreign exchange gain on forward contracts                              | (10,961 )   | (16,491 )    |
| Non-cash changes in the fair value of contingent acquisition consideration payable | (56,954 )   | 15,101       |
| Other  | 1,044       | 1,059        |
| Changes in operating assets and liabilities:                                       |             |              |
| Accounts receivable, net   | (52,023 )   | 146          |
| Inventory  | (59,802 )   | (47,469 )    |
| Other current assets   | (1,556 )    | (27,970 )    |
| Other assets   | (5,002 )    | (1,391 )     |
| Accounts payable and accrued liabilities   | (77,852 )   | 924          |
| Other long-term liabilities  | (4,451 )    | 790          |
| Net cash used in operating activities  | (228,190)   | (153,652 )   |
| <b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>                                       |             |              |
| Purchases of property, plant and equipment   | (96,806 )   | (123,844 )   |
| Funds held in escrow for the purchase of real property                             | (8,383 )    | (12,500 )    |
| Maturities and sales of investments  | 302,801     | 261,786      |
| Purchase of available-for-sale securities  | (370,393)   | (842,873 )   |
| Purchase of promissory note  | —           | (3,326 )     |
| Business acquisitions, net of cash acquired  | (1,467 )    | (538,392 )   |
| Other  | (150 )      | —            |
| Net cash used in investing activities  | (174,398)   | (1,259,149)  |
| <b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>                                       |             |              |
| Proceeds from exercises of stock options and the ESPP                              | 49,498      | 51,515       |
| Taxes paid related to net share settlement of equity awards                        | (55,241 )   | (21,968 )    |
| Proceeds from public offering of common stock, net                                 | 712,938     | 888,257      |
| Excess tax benefit from stock option exercises                                     | 563         | 463          |
| Other  | —           | (2,062 )     |
| Net cash provided by financing activities  | 707,758     | 916,205      |

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|  |           |            |
|--|-----------|------------|
| Effect of exchange rate changes on cash  | 5,139     | (2,544 )   |
| NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS                                 | 310,309   | (499,140 ) |
| Cash and cash equivalents:   |           |            |
| Beginning of period  | \$397,040 | \$875,486  |
| End of period  | \$707,349 | \$376,346  |
| SUPPLEMENTAL CASH FLOW DISCLOSURES:  |           |            |
| Cash paid for interest, net of interest capitalized into fixed assets                | 4,564     | 4,979      |
| Cash paid for income taxes   | 95,163    | 15,377     |
| Stock-based compensation capitalized into inventory                                  | 8,960     | 8,271      |
| Depreciation capitalized into inventory  | 13,402    | 11,005     |
| SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON CASH INVESTING AND FINANCING              |           |            |
| ACTIVITIES:  |           |            |
| Decrease in accounts payable and accrued liabilities related to fixed assets         | (13,988 ) | (11,386 )  |
| Conversion of convertible debt   | 8,924     | 8,957      |
| Accrual for inventory purchases related to the acquisition of the Merck PKU Business | 1,322     | —          |

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

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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

(1) NATURE OF OPERATIONS AND BUSINESS RISKS

BioMarin Pharmaceutical Inc. (the Company), a Delaware corporation, develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The Company 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 consists of five approved products and multiple clinical and investigational product candidates. The Company's approved products are Vimizim (elosulfase alfa), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

The Company expects to continue to finance future cash needs that exceed its operating activities primarily through its current cash, cash equivalents, short-term and long-term investments and, to the extent appropriate, through proceeds from equity or debt financings, loans and collaborative agreements with corporate partners. Until the Company consistently generates positive cash flows from its operations, the Company expects to raise the capital necessary to fund its current operations and long-term plans. Additional capital may also be necessary if the Company enters into potential licenses and other acquisitions of complementary technologies, products or companies.

The Company is subject to a number of risks and uncertainties, including, without limitation: the financial performance of its approved products; the expected need for additional financings; the Company's ability to successfully commercialize its approved products; the uncertainty of the Company's research and development (R&D) efforts resulting in future successful commercial products; the Company's ability to successfully obtain regulatory approval for new products; the Company's ability to compete effectively; 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 the United States generally accepted accounting principles (U.S. GAAP) for complete financial statements, although the Company believes that the disclosures herein are adequate to ensure that the information presented is not misleading. The Condensed Consolidated Financial Statements should therefore be read in conjunction with the Consolidated Financial Statements and Notes thereto for the fiscal year ended December 31, 2015 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, 2016 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2016 or any other period.

Management performed an evaluation of the Company's activities through the date of filing of this Quarterly Report on Form 10-Q, and has concluded that there were no subsequent events or transactions that occurred subsequent to the balance sheet date prior to filing this Quarterly Report on Form 10-Q that would require recognition or disclosure in the Condensed Consolidated Financial Statements.

### (3) SIGNIFICANT ACCOUNTING POLICIES

There have been no material changes to the Company's significant accounting policies during the nine months ended September 30, 2016, 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, 2015.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

(4) RECENT ACCOUNTING PRONOUNCEMENTS

Except as described below, there have been no new accounting pronouncements or changes to accounting pronouncements during the nine months ended September 30, 2016, as compared to the recent accounting pronouncements described in Note 4 of the Company's Annual Report on Form 10-K for the year ended December 31, 2015, that are of significance or potential significance to the Company.

In March 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2016-09, Improvements to Employee Share-Based Payment Accounting (ASU 2016-09), which is intended to simplify several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. ASU 2016-09 is effective for fiscal years beginning after December 15, 2016, including interim periods within those fiscal years. Early application is permitted. ASU 2016-09 will be effective for the Company's fiscal year beginning January 1, 2017 unless it elects early adoption. The Company is currently evaluating the potential impact the adoption of ASU 2016-09 will have on its consolidated financial statements and as of September 30, 2016, the Company has not made the election to early adopt the amendments.

In February 2016, the FASB issued ASU No. 2016-02, Leases (ASU 2016-02). The amended guidance requires balance sheet recognition of lease assets and liabilities by lessees for leases classified as operating leases, with an option to not recognize lease assets and lease liabilities for leases with a term of 12 months or less. The amendments also require new disclosures providing additional qualitative and quantitative information about the amounts recorded in the financial statements. Lessor accounting is largely unchanged. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. Early application is permitted. ASU 2016-02 will be effective for the Company's fiscal year beginning January 1, 2019 unless it elects early adoption. The amendments require a modified retrospective approach with optional practical expedients. The Company is currently evaluating the potential impact the adoption of ASU 2016-02 will have on its consolidated financial statements and has not made the election to early adopt ASU 2016-02.

In May 2014, the FASB issued ASU No. 2014-09 (ASU 2014-09) regarding Accounting Standards Codification (ASC) Topic 606, Revenue from Contracts with Customers. ASU 2014-09 provides principles for recognizing revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the Company expects to be entitled in exchange for those goods or services. In August 2015, the FASB issued ASU No. 2015-14 to defer the effective date by one year with early adoption permitted as of the original effective date. ASU 2014-09 will be effective for the Company's fiscal year beginning January 1, 2018 unless it elects the earlier date of January 1, 2017. In March 2016, the FASB issued ASU No. 2016-08 to help provide interpretive clarifications on the new guidance for ASC Topic 606. In April 2016, the FASB issued ASU No. 2016-10 to clarify the guidance for identifying performance obligations and accounting for licenses of intellectual property. In May 2016, the FASB issued ASU No. 2016-12 to improve the guidance on collectibility, noncash consideration, and completed contracts at transition. The Company is currently evaluating the accounting, transition, and disclosure requirements of the standard.

(5) ACQUISITIONS

The Merck PKU Business

On October 1, 2015, the Company entered into a Termination and Transition Agreement with Ares Trading S.A. (Merck Serono), as amended and restated on December 23, 2015 (the A&R Kuvan Agreement), to terminate the Development, License and Commercialization Agreement, dated May 13, 2005, as amended (the License Agreement), between the Company and Merck Serono, including the license to Kuvan the Company had granted to Merck Serono under the License Agreement. Also on October 1, 2015, the Company and Merck Serono entered into a Termination Agreement (the Pegvaliase Agreement) to terminate the license to pegvaliase the Company had granted to Merck Serono under the License Agreement. On January 1, 2016, pursuant to the A&R Kuvan Agreement and the Pegvaliase Agreement, the Company completed the acquisition from Merck Serono and its affiliates of certain rights and other assets with respect to Kuvan and pegvaliase (the Merck PKU Business). As a result, the Company acquired all global rights to Kuvan and pegvaliase from Merck Serono, with the exception of Kuvan in Japan. Previously, the Company had exclusive rights to Kuvan in the United States (U.S.) and Canada and pegvaliase in the U.S. and Japan. In connection with the acquisition of the Merck PKU Business, the Company recognized transaction costs of \$0.6 million, of which \$0.3 million was recognized in each of the year ended December 31, 2015 and in the nine month period ended September 30, 2016.

## BIOMARIN PHARMACEUTICAL INC.

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

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

Pursuant to the A&R Kuvan Agreement, the Company paid Merck Serono \$374.5 million, in cash, the majority of which was paid in January 2016, and is obligated to pay Merck Serono up to a maximum of €60.0 million, in cash, if future sales milestones are met. Pursuant to the Pegvaliase Agreement, the Company is obligated to pay Merck Serono up to a maximum of €125.0 million, in cash, if future development milestones are met. Merck Serono transferred certain inventory, regulatory materials and approvals, and intellectual property rights to the Company and will perform certain transition services for the Company.

The Company and Merck Serono have no further rights or obligations under the License Agreement with respect to pegvaliase. The License Agreement will continue in effect, but in no event later than December 31, 2016, in order to complete the transfer of certain assets related to Kuvan, the majority of which occurred in January 2016. Accordingly, as of September 30, 2016, the Company continues to rely on Merck Serono to provide critical transition services for the sales and distribution of Kuvan in approximately eight remaining countries until marketing authorizations can be transferred in such countries.

Prior to the consummation of the transactions described above, the Company sold Kuvan to Merck Serono at a price near its manufacturing costs, and Merck Serono resold the product to end users outside the U.S., Canada and Japan. The royalty earned by the Company from Kuvan product sold by Merck Serono was included as a component of Net Product Revenues in the period earned.

Kuvan is a commercialized product for the treatment of patients with phenylketonuria (PKU) and/or for primary BH4 deficiency in certain countries. Pegvaliase is currently in pivotal studies as a potential therapeutic option for adult patients with PKU. In March 2016, the Company announced that its pivotal Phase 3 PRISM-2 study of pegvaliase met the primary endpoint of change in blood Phe compared with placebo ( $p < 0.0001$ ); and the Company also announced its plans to submit a marketing application in the U.S. Kuvan has Orphan Drug exclusivity in the European Union (EU) until 2020, and pegvaliase has Orphan Drug designation in the U.S. and the EU.

The acquisition date fair value of the contingent acquisition consideration payments, Kuvan global marketing rights, with the exception of Japan, and pegvaliase in-process research and development (IPR&D) acquired was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as level 3 inputs. Key assumptions include a discount rate and various probability factors. The range of outcomes and assumptions used to develop these estimates has been updated to estimate the fair value of the contingent acquisition consideration payable at September 30, 2016. See Note 13 to these Condensed Consolidated Financial Statements for additional discussion regarding fair value measurements of the contingent acquisition consideration payable included on the Company's Condensed Consolidated Balance Sheet.

The following table presents the preliminary allocation of the purchase consideration for the Merck PKU Business acquisition, including the contingent acquisition consideration payable based on the acquisition date fair value. The allocation of the purchase price below reflects an inventory adjustment in the second quarter of 2016.

|               |           |
|---------------|-----------|
| Cash payments | \$374,545 |
|---------------|-----------|



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|  |           |
|--|-----------|
| Estimated fair value of contingent acquisition consideration payable | 138,974   |
| Total consideration  | \$513,519 |

|                                    |           |
|------------------------------------|-----------|
| Kuvan intangible assets            | \$172,961 |
| Pegvaliase IPR&D                   | 326,359   |
| Inventory                          | 14,199    |
| Total identifiable assets acquired | \$513,519 |

The amount allocated to the Kuvan intangible assets is considered to be finite-lived and will be amortized on a straight-line basis over its estimated useful life through 2024.

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## BIOMARIN PHARMACEUTICAL INC.

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

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

The amount allocated to acquired pegvaliase IPR&D is 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 the reduction in the fair value of the IPR&D assets below their respective carrying amounts. When development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point. See Note 8 to these Condensed Consolidated Financial Statements for further discussion of the indefinite-lived intangible assets.

## Pro Forma Financial Information

The following unaudited pro forma financial information presents the combined results of operations of the Company and the Merck PKU Business as if the acquisition occurred on January 1, 2015. This unaudited pro forma financial information is presented for informational purposes only and is not necessarily indicative of the results of future operations that would have been achieved had the acquisitions taken place at the beginning of 2015.

|   | Three Months<br>Ended<br>September 30,<br>2015 | Nine Months<br>Ended<br>September 30,<br>2015 |
|---|--|---|
| Total revenues                                      | \$ 225,697                                     | \$ 713,930                                    |
| Net loss  | \$ (84,395 )                                   | \$ (220,465 )                                 |
| Net loss per share, basic                           | \$ (0.52 )                                     | \$ (1.38 )                                    |
| Net loss per share, diluted                         | \$ (0.55 )                                     | \$ (1.38 )                                    |
| Weighted average common shares outstanding, basic   | 160,886  | 159,647                                       |
| Weighted average common shares outstanding, diluted | 161,134  | 159,647                                       |

## (6) NET 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 ESPP, unvested restricted stock units (RSUs), common stock held by the NQDC 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 (in thousands of common shares):

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|   | Three Months Ended |             | Nine Months Ended |              |
|---|--------------------|-------------|-------------------|--------------|
|   | September 30,      |             | September 30,     |              |
|   | 2016               | 2015        | 2016              | 2015         |
| <b>Numerator:</b>                                   |                    |             |                   |              |
| Net loss, basic                                     | \$(42,797 )        | \$(90,926 ) | \$(551,499 )      | \$(240,416 ) |
| Gain on common stock held by the NQDC               | —                  | (4,980 )    | (1,753 )          | —            |
| Net loss, diluted                                   | \$(42,797 )        | \$(95,906 ) | \$(553,252 )      | \$(240,416 ) |
| <b>Denominator:</b>                                 |                    |             |                   |              |
| Weighted-average common shares outstanding, basic   | 167,714            | 160,886     | 163,963           | 159,647      |
| <b>Effect of dilutive securities:</b>               |                    |             |                   |              |
| Common shares held by the NQDC                      | —                  | 248         | 253               | —            |
| Weighted-average common shares outstanding, diluted | 167,714            | 161,134     | 164,216           | 159,647      |
| Net loss per common share, basic                    | \$(0.26 )          | \$(0.57 )   | \$(3.36 )         | \$(1.51 )    |
| Net loss per common share, diluted                  | \$(0.26 )          | \$(0.60 )   | \$(3.37 )         | \$(1.51 )    |

## BIOMARIN PHARMACEUTICAL INC.

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

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

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

|  | Three Months<br>Ended<br>September 30,<br>2016 |        | Nine Months<br>Ended<br>September 30,<br>2015 |        |
|--|--|--------|---|--------|
| Options to purchase common stock                     | 9,610  | 10,503 | 9,610   | 10,503 |
| Common stock issuable under the 2017 Notes           | 1,105  | 1,553  | 1,105   | 1,553  |
| Common stock issuable under the 2018 and 2020 Notes  | 7,966  | 7,966  | 7,966   | 7,966  |
| Unvested restricted stock units                      | 2,728  | 1,757  | 2,728   | 1,633  |
| Common stock potentially issuable for ESPP purchases | 130  | 229    | 114   | 220    |
| Common stock held by the NQDC                        | 253  | —      | —   | 248    |
| Total number of potentially issuable shares          | 21,792   | 22,008 | 21,523  | 22,123 |

The effect of the Company's 0.7% senior subordinated convertible notes due in 2018 (the 2018 Notes) and the Company's 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes, and together with the 2018 Notes, the Notes) were excluded from the diluted net loss per common share because they were antidilutive. The Company's closing stock price on September 30, 2016 and 2015 did not exceed the conversion price of \$94.15 per share for the Notes.

## (7) INVESTMENTS

All investments were classified as available-for-sale at September 30, 2016 and December 31, 2015. 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, 2016 and December 31, 2015 are summarized in the tables below:

|                   | Gross<br>Unrealized | Gross<br>Unrealized | Aggregate Fair<br>Value at<br>September 30,<br>2016 |
|-------------------|---------------------|---------------------|---|
| Amortized<br>Cost | Holding<br>Gains    | Holding<br>Losses   |   |

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|                                   |            |          |           |            |
|-----------------------------------|------------|----------|-----------|------------|
| Certificates of deposit           | \$ 2,800   | \$ —     | \$ —      | \$ 2,800   |
| Corporate debt securities         | 420,259    | 873      | (165 )    | 420,967    |
| Commercial paper                  | 16,692     | —        | —         | 16,692     |
| U.S. government agency securities | 249,674    | 190      | (2 )      | 249,862    |
| Greek government-issued bonds     | 52         | 82       | —         | 134        |
| Total                             | \$ 689,477 | \$ 1,145 | \$ (167 ) | \$ 690,455 |

|                                   | Amortized Cost | Gross Unrealized Holding Gains | Gross Unrealized Holding Losses | Aggregate Fair Value at December 31, 2015 |
|-----------------------------------|----------------|--------------------------------|---------------------------------|---|
| Certificates of deposit           | \$ 63,919      | \$ 1                           | \$ —                            | \$ 63,920                                 |
| Corporate debt securities         | 358,625        | 20                             | (732 )                          | 357,913                                   |
| Commercial paper                  | 12,733         | —                              | —                               | 12,733                                    |
| U.S. government agency securities | 186,882        | —                              | (344 )                          | 186,538                                   |
| Greek government-issued bonds     | 48             | 79                             | —                               | 127                                       |
| Total                             | \$ 622,207     | \$ 100                         | \$ (1,076 )                     | \$ 621,231                                |

As of December 31, 2015, the Company had two investments in marketable equity securities measured using quoted prices in their respective active markets that are collectively considered strategic investments. In the second quarter of 2016, the remaining shares of one strategic investment were sold for a realized loss of \$2.0 million. As of September 30, 2016, the fair value of the Company's marketable equity securities was \$7.3 million, which included an unrealized gain of \$4.3 million. As of December 31, 2015, the fair value of the Company's marketable equity securities was \$18.1 million, which included an unrealized gain of \$12.7 million. The Company's investments are recorded in Other Assets in the Company's Condensed Consolidated Balance Sheets.

## 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 of available-for-sale securities by contractual maturity were as follows:

|  | September 30,<br>2016 | December 31,<br>2015 |
|--|-----------------------|----------------------|
| Maturing in one year or less               | \$ 327,499            | \$ 195,579           |
| Maturing after one year through five years | 362,956               | 425,652              |
| Total                                      | \$ 690,455            | \$ 621,231           |

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, 2016, 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 13 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of the Company's available-for-sale securities.

## (8) INTANGIBLE ASSETS

Intangible assets consisted of the following:

|                                    | September 30,<br>2016 | December 31,<br>2015 |
|------------------------------------|-----------------------|----------------------|
| Intangible assets:                 |                       |                      |
| Finite-lived intangible assets     | \$ 305,122            | \$ 129,572           |
| Indefinite-lived intangible assets | 332,199               | 607,548              |
| Gross intangible assets:           | 637,321               | 737,120              |
| Less: Accumulated amortization     | (75,934 )             | (53,124 )            |
| Net carrying value                 | \$ 561,387            | \$ 683,996           |

Indefinite-Lived Intangible Assets

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

During the second quarter of 2016, the Company withdrew its Marketing Authorization Application (MAA) for Kyndrisa from the European Medicines Agency (EMA) and announced it would discontinue clinical and regulatory development of Kyndrisa as well as the three other first-generation follow-on products, BMN 044, BMN 045 and BMN 053 (other exons), for the treatment of distinct forms of Duchenne muscular dystrophy. Based on the then current status of the European development efforts, the Company recognized an impairment charge of \$574.1 million in the second quarter of 2016 related to the Kyndrisa and other exon IPR&D assets reducing the remaining book value to zero. The Company also recognized an impairment charge of \$25.0 million in the second quarter of 2016 related to the reveglucosidase alfa IPR&D assets due to the decision to terminate that development program. No intangible asset impairment charges were recognized during the three and nine months ended September 30, 2015.

See Note 8 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015 for additional information related to the Company's intangible assets.

## BIOMARIN PHARMACEUTICAL INC.

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

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

## (9) PROPERTY, PLANT AND EQUIPMENT

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

|  | September 30,<br>2016 | December 31,<br>2015 |
|--|-----------------------|----------------------|
| Building and improvements                | \$ 505,894            | \$ 442,100           |
| Manufacturing and laboratory equipment   | 229,818               | 145,313              |
| Computer hardware and software           | 123,557               | 113,442              |
| Leasehold improvements                   | 44,802                | 44,247               |
| Furniture and equipment                  | 26,359                | 22,817               |
| Land improvements                        | 4,881                 | 4,881                |
| Land                                     | 45,727                | 45,727               |
| Construction-in-progress                 | 76,992                | 164,283              |
|  | 1,058,030             | 982,810              |
| Less: Accumulated depreciation           | (328,194 )            | (278,603 )           |
| Total property, plant and equipment, net | \$ 729,836            | \$ 704,207           |

Construction in-process primarily includes costs related to the Company's significant in-process projects at its facilities in Marin County, California, and its manufacturing facility in Shanbally, Cork, Ireland.

Depreciation expense for the three and nine months ended September 30, 2016 was \$23.2 million and \$56.1 million, respectively, of which \$4.4 million and \$13.4 million, respectively, was capitalized into inventory. Depreciation expense for the three and nine months ended September 30, 2015 was \$12.7 million and \$36.4 million, respectively, of which \$3.7 million and \$11.0 million, respectively, was capitalized into inventory. Capitalized interest related to the Company's property, plant and equipment purchases for each of the three and nine months ended September 30, 2016 and 2015 was insignificant.

## (10) SUPPLEMENTAL BALANCE SHEET INFORMATION

Inventory consisted of the following:

|                 | September 30,<br>2016 | December 31,<br>2015 |
|-----------------|-----------------------|----------------------|
| Raw materials   | \$ 46,951             | \$ 46,115            |
| Work-in-process | 189,596               | 150,289              |



|                 |            |            |
|-----------------|------------|------------|
| Finished goods  | 110,873    | 75,279     |
| Total inventory | \$ 347,420 | \$ 271,683 |

## BIOMARIN PHARMACEUTICAL INC.

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

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

In the first quarter of 2016, process qualification production activities commenced in the Company's Shanbally facility related to Vimizim production. As of September 30, 2016, the value of the qualification campaign was \$29.8 million, which was capitalized as inventory because the product is expected to be sold commercially. While the Company believes it is unlikely that the manufacturing process will not be approved for Vimizim production, should that occur, the value of the inventory would be expensed at that time.

Inventory as of September 30, 2016, included \$31.3 million of pre-launch Brineura (formerly referred to as cerliponase alfa) inventory for production that commenced in the second quarter of 2016. Brineura is an investigational therapy to treat children with CLN2 disease, or late infantile neuronal ceroid lipofuscinosis, a lysosomal storage disorder primarily affecting the brain. The Company must receive marketing approval from the applicable regulators before the Brineura inventory can be sold commercially. Although regulatory approval cannot be assured, the Company expects to receive regulatory approval and realize the costs of the inventory through future sales. The Company believes that all material uncertainties related to the ultimate regulatory approval of Brineura for commercial sale have been significantly reduced based on positive data from Phase I/II clinical trial results and the filings of Biologics License Application (BLA) with the Food and Drug Administration (FDA) and the MAA with the EMA during the second quarter of 2016. In its evaluation, the Company also considered its historical experience with developing and commercially producing similar products for rare genetic disorders.

Other Assets consisted of the following:

|   | September 30,<br>2016 | December 31,<br>2015 |
|---|-----------------------|----------------------|
| Deposit for business acquisition                            | \$ —                  | \$ 371,756           |
| Deposits  | 19,748                | 8,606                |
| Strategic investments                                       | 7,267                 | 18,056               |
| Long-term forward foreign currency exchange contract assets | 750                   | 3,533                |
| Other   | 8,678                 | 6,693                |
| Total other assets  | \$ 36,443             | \$ 408,644           |

Included in Deposits is an \$8.4 million escrow deposit to purchase land in Shanbally, Ireland.

Accounts Payable and Accrued Liabilities consisted of the following:

|   | September 30,<br>2016 | December 31,<br>2015 |
|---|-----------------------|----------------------|
| Accounts payable and accrued operating expenses | \$ 148,773            | \$ 179,294           |
| Accrued compensation expense                    | 85,219                | 95,345               |
| Accrued rebates payable                         | 34,184                | 32,553               |
| Accrued royalties payable                       | 14,183                | 10,412               |
| Value added taxes payable                       | 7,961                 | 6,377                |

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|  |            |            |
|--|------------|------------|
| Accrued income taxes                           | —          | 59,572     |
| Other  | 15,809     | 8,958      |
| Total accounts payable and accrued liabilities | \$ 306,129 | \$ 392,511 |

Accounts payable and accrued operating expenses as of September 30, 2016, included approximately \$4.9 million of employee termination benefits accrued as a result of the termination of the Kyndrisa and other exon development programs. This plan was approved and communicated to the impacted employees in the third quarter of 2016. Cash payments to employees will be made through the first quarter of 2017.

## BIOMARIN PHARMACEUTICAL INC.

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

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

## (11) CONVERTIBLE DEBT

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

|  | September 30, 2016 | December 31, 2015 |
|--|--------------------|-------------------|
| Convertible Notes due in 2017                | \$22,506           | \$31,430          |
| Unamortized deferred offering costs          | (46 )              | (110 )            |
| Convertible Notes due in 2017, net           | 22,460             | 31,320            |
| Convertible Notes due in 2018                | 374,980            | 374,980           |
| Unamortized discount                         | (31,219 )          | (41,904 )         |
| Unamortized deferred offering costs          | (3,968 )           | (5,415 )          |
| Convertible Notes due in 2018, net           | 339,793            | 327,661           |
| Convertible Notes due in 2020                | 374,993            | 374,993           |
| Unamortized discount                         | (56,364 )          | (65,478 )         |
| Unamortized deferred offering costs          | (5,244 )           | (6,210 )          |
| Convertible Notes due in 2020, net           | 313,385            | 303,305           |
| Total convertible debt, net                  | \$675,638          | \$662,286         |
| Fair value of fixed rate convertible debt    |                    |                   |
| Convertible Notes due in 2017 <sup>(1)</sup> | \$101,789          | \$162,016         |
| Convertible Notes due in 2018 <sup>(1)</sup> | 448,247            | 482,584           |
| Convertible Notes due in 2020 <sup>(1)</sup> | 469,487            | 502,701           |
| Total  | \$1,019,523        | \$1,147,301       |

(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 consisted of the following:

|  | Three Months Ended |                    | Nine Months Ended  |                    |
|--|--------------------|--------------------|--------------------|--------------------|
|  | September 30, 2016 | September 30, 2015 | September 30, 2016 | September 30, 2015 |
| Coupon interest                            | \$2,466            | \$2,283            | \$7,491            | \$7,667            |
| Amortization of issuance costs             | 826                | 823                | 2,476              | 2,471              |
| Accretion of debt discount                 | 6,688              | 6,341              | 19,800             | 18,773             |
| Total interest expense on convertible debt | \$9,980            | \$9,447            | \$29,767           | \$28,911           |

During the three and nine months ended September 30, 2016, certain existing holders of the Company's senior subordinated notes due in 2017 (the 2017 Notes) elected to convert \$2.0 million and \$8.9 million, respectively, in aggregate principal amount of the 2017 Notes into 97,348 and 438,315 shares of the Company's common stock, respectively. During the nine months ended September 30, 2015, the Company entered into three separate agreements with certain existing holders of its 2017 Notes pursuant to which such holders converted \$8.1 million in aggregate principal amount of the 2017 Notes into 399,469 shares of the Company's common stock. In addition to issuing the requisite number of shares of the Company's common stock, the Company also made varying cash payments to the holders totaling \$0.2 million in the aggregate, of which \$0.2 million was recognized in total as debt conversion expense on the Company's Condensed Consolidated Statement of Comprehensive Loss for the nine months ended September 30, 2015.

See Note 14 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015 for additional information related to the Company's convertible debt.

## BIOMARIN PHARMACEUTICAL INC.

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

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

## (12) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

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

The Company designates certain of these forward foreign currency exchange contracts as hedging instruments and enters into some forward foreign currency exchange contracts that are considered to be economic hedges that are not designated as hedging instruments. Whether designated or undesignated, these forward foreign currency exchange contracts protect against the reduction in value of forecasted foreign currency cash flows resulting from product revenues, royalty revenues, operating expenses and asset or liability positions designated in currencies other than the U.S. dollar. The fair values of forward foreign currency exchange contracts are estimated using current exchange rates and interest rates, and take into consideration the current creditworthiness of the counterparties or the Company, as applicable. Information regarding the specific instruments used by the Company to hedge its exposure to foreign currency exchange rate fluctuations is provided below. See Note 13 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of forward foreign currency exchange contracts.

The Company enters into forward foreign currency exchange contracts in order to protect against the fluctuations in revenue and operating expenses associated with foreign currency-denominated cash flows. The Company has formally designated these forward foreign currency exchange contracts as cash flow hedges and expects them to be highly effective in offsetting fluctuations in operating expenses denominated in Euros and revenues denominated in currencies other than the U.S. dollar related to changes in foreign currency exchange rates.

The following table summarizes the Company's designated forward foreign currency exchange contracts outstanding as of September 30, 2016 (notional amounts in millions):

| Foreign Exchange Contracts | Number<br>of<br>Contracts | Aggregate<br>Notional<br>Amount<br>in<br>Foreign<br>Currency | Maturity              |
|----------------------------|---------------------------|--|-----------------------|
| Euros - Purchase           | 84                        | 92.5   | Oct. 2016 - Sep. 2019 |
| Euros - Sell               | 280                       | 316.5  | Oct. 2016 - Sep. 2019 |
| Canadian Dollars - Sell    | 6                         | 3.1  | Oct. 2016 - Dec. 2016 |
| Colombian Pesos - Sell     | 3                         | 9,798.0  | Oct. 2016 - Dec. 2016 |
| British Pounds - Sell      | 1                         | 0.6  | October 2016          |
| Total                      | 374                       |  |                       |

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 selling, general and administrative (SG&A) expense in the Company's Condensed Consolidated Statements of Comprehensive Loss.

The following table summarizes the Company's non-designated forward foreign currency exchange contracts outstanding as of September 30, 2016 (notional amounts in millions):

| Foreign Exchange Contracts | Number<br>of<br>Contracts | Aggregate<br>Notional<br>Amount<br>in<br>Foreign<br>Currency | Maturity     |
|----------------------------|---------------------------|--|--------------|
| Euros - Purchase           | 1                         | 99.4   | October 2016 |
| British Pounds - Sell      | 1                         | 6.9  | October 2016 |
| Total                      | 2                         |  |              |

## 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 maximum length of time over which the Company is hedging its exposure to the reduction in value of forecasted foreign currency revenues through forward foreign currency exchange contracts is through September 2019. Over the next twelve months, the Company expects to reclassify \$0.5 million from accumulated other comprehensive income to earnings as the forecasted revenue and operating expense transactions occur.

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

|  | Asset Derivatives<br>September 30, 2016 |            | Liability Derivatives<br>September 30, 2016 |            |
|--|---|------------|---|------------|
|  | Balance Sheet Location                  | Fair Value | Balance Sheet Location                      | Fair Value |
| Derivatives designated as hedging instruments:     |   |            |   |            |
| Forward foreign currency exchange contracts        | Other current assets                    | \$ 4,785   | Accounts payable and accrued liabilities    | \$ 3,290   |
| Forward foreign currency exchange contracts        | Other assets                            | 750        | Other long- term liabilities                | 1,979      |
| Total  |   | 5,535      |   | 5,269      |
| Derivatives not designated as hedging instruments: |   |            |   |            |
| Forward foreign currency exchange contracts        | Other current assets                    | 25         | Accounts payable and accrued liabilities    | 65         |
| Forward foreign currency exchange contracts        | Other assets                            | —          | Other long- term liabilities                | —          |
| Total  |   | 25         |   | 65         |
| Total value of derivative contracts                |   | \$ 5,560   |   | \$ 5,334   |

|  | Asset Derivatives<br>December 31, 2015 |            | Liability Derivatives<br>December 31, 2015 |            |
|--|--|------------|--|------------|
|  | Balance Sheet Location                 | Fair Value | Balance Sheet Location                     | Fair Value |
| Derivatives designated as hedging instruments:     |  |            |  |            |
| Forward foreign currency exchange contracts        | Other current assets                   | \$ 10,478  | Accounts payable and accrued liabilities   | \$ 1,986   |
| Forward foreign currency exchange contracts        | Other assets                           | 3,533      | Other long- term liabilities               | 3,057      |
| Total  |  | 14,011     |  | 5,043      |
| Derivatives not designated as hedging instruments: |  |            |  |            |
| Forward foreign currency exchange contracts        | Other current assets                   | —          | Accounts payable and accrued liabilities   | 22         |



|                                     |           |          |
|-------------------------------------|-----------|----------|
| Total                               | —         | 22       |
| Total value of derivative contracts | \$ 14,011 | \$ 5,065 |
| 17                                  |           |          |

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## BIOMARIN PHARMACEUTICAL INC.

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

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

The effect of the Company's derivative instruments on the Condensed Consolidated Financial Statements for the three and nine months ended September 30, 2016 and 2015 was as follows:

|   | Three Months<br>Ended<br>September 30,<br>2016 |          | Nine Months<br>Ended<br>September 30,<br>2015 |          |
|---|--|----------|---|----------|
| <b>Derivatives Designated as Hedging Instruments:</b>                         |  |          |   |          |
| Net gain (loss) recognized in Other Comprehensive Income (OCI) <sup>(1)</sup> | \$(1,984)                                      | \$3,126  | \$(5,857)                                     | \$14,191 |
| Net gain reclassified from accumulated OCI into earnings <sup>(2)</sup>       | 1,486  | 5,187    | 4,616   | 15,084   |
| Net gain (loss) recognized in net loss <sup>(3)</sup>                         | 9  | (264 )   | 5,276   | (404 )   |
| <b>Derivatives Not Designated as Hedging Instruments:</b>                     |  |          |   |          |
| Net gain (loss) recognized in net loss <sup>(4)</sup>                         | \$826  | \$(514 ) | \$(2,446)                                     | \$6,052  |

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

(2) Effective portion classified as Net Product Revenues and SG&A expense.

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

(4) Classified as SG&A expense.

At September 30, 2016 and December 31, 2015, accumulated other comprehensive income associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a gain of \$1.2 million and \$13.6 million, respectively.

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

## BIOMARIN PHARMACEUTICAL INC.

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

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

## (13) FAIR VALUE MEASUREMENTS

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

| Fair Value Measurements at September 30, 2016             |  |  |  |                |
|---|--|--|--|----------------|
| Quoted Price in   |  |  |  |                |
|   | Active<br>Markets<br>For<br>Identical<br>Assets<br>(Level 1) | Significant Other<br>Observable<br>Inputs<br>(Level 2) | Significant<br>Unobservable<br>Inputs<br>(Level 3) | Total          |
| <b>Assets:</b>  |  |  |  |                |
| <b>Cash and cash equivalents:</b>                         |  |  |  |                |
| Overnight deposits  | \$246,568  | \$ —   | \$ —   | \$246,568      |
| Money market instruments                                  | —  | 460,781  | —  | 460,781        |
| <b>Total cash and cash equivalents</b>                    | <b>246,568</b>   | <b>460,781</b>   | <b>—</b>   | <b>707,349</b> |
| <b>Available-for-sale securities:</b>                     |  |  |  |                |
| <b>Short-term:</b>  |  |  |  |                |
| Certificates of deposit                                   | —  | 2,800  | —  | 2,800          |
| Corporate debt securities                                 | —  | 139,073  | —  | 139,073        |
| Commercial paper  | —  | 16,692   | —  | 16,692         |
| U.S. government agency securities                         | —  | 168,934  | —  | 168,934        |
| <b>Long-term:</b>   |  |  |  |                |
| Corporate debt securities                                 | —  | 281,894  | —  | 281,894        |
| U.S. government agency securities                         | —  | 80,928   | —  | 80,928         |
| Greek government-issued bonds                             | —  | 134  | —  | 134            |
| <b>Total available-for-sale securities</b>                | <b>—</b>   | <b>690,455</b>   | <b>—</b>   | <b>690,455</b> |
| <b>Other current assets:</b>                              |  |  |  |                |
| Nonqualified Deferred Compensation Plan assets            | —  | 309  | —  | 309            |
| Forward foreign currency exchange contract <sup>(1)</sup> | —  | 4,810  | —  | 4,810          |
| Restricted investments <sup>(2)</sup>                     | —  | 4,052  | —  | 4,052          |
| <b>Total other current assets</b>                         | <b>—</b>   | <b>9,171</b>   | <b>—</b>   | <b>9,171</b>   |
| <b>Other assets:</b>                                      |  |  |  |                |
| Nonqualified Deferred Compensation Plan assets            | —  | 8,562  | —  | 8,562          |

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|   |           |              |            |             |
|---|-----------|--------------|------------|-------------|
| Forward foreign currency exchange contract <sup>(1)</sup> | —         | 750          | —          | 750         |
| Strategic investment <sup>(3)</sup>                       | 7,267     | —            | —          | 7,267       |
| Total other assets  | 7,267     | 9,312        | —          | 16,579      |
| Total assets  | \$253,835 | \$ 1,169,719 | \$ —       | \$1,423,554 |
| <b>Liabilities:</b>                                       |           |              |            |             |
| <b>Current liabilities:</b>                               |           |              |            |             |
| Nonqualified Deferred Compensation Plan liability         | \$4,008   | \$ 309       | \$ —       | \$4,317     |
| Forward foreign currency exchange contract <sup>(1)</sup> | —         | 3,355        | —          | 3,355       |
| Contingent acquisition consideration payable              | —         | —            | 48,746     | 48,746      |
| Total current liabilities                                 | 4,008     | 3,664        | 48,746     | 56,418      |
| <b>Other long-term liabilities:</b>                       |           |              |            |             |
| Nonqualified Deferred Compensation Plan liability         | \$19,394  | \$ 8,562     | —          | 27,956      |
| Forward foreign currency exchange contract <sup>(1)</sup> | —         | 1,979        | —          | 1,979       |
| Contingent acquisition consideration payable              | —         | —            | 122,644    | 122,644     |
| Total other long-term liabilities                         | 19,394    | 10,541       | 122,644    | 152,579     |
| Total liabilities   | \$23,402  | \$ 14,205    | \$ 171,390 | \$208,997   |

## 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, 2015                         |  |  | Total        |  |  |  |  |
|---|--|--|--|--------------|--|--|--|--|
|   | Quoted Price in  |  |  |              |  |  |  |  |
|   | Active<br>Markets<br><br>For<br>Identical<br>Assets<br><br>(Level 1) | Significant Other<br>Observable<br>Inputs<br><br>(Level 2) | Significant<br>Unobservable<br>Inputs<br><br>(Level 3) |              |  |  |  |  |
|   |  |  |  |              |  |  |  |  |
|   |  |  |  |              |  |  |  |  |
| <b>Assets:</b>  |  |  |  |              |  |  |  |  |
| <b>Cash and cash equivalents:</b>                         |  |  |  |              |  |  |  |  |
| Overnight deposits  | \$ 290,731   | \$ —   | \$ —   | \$ 290,731   |  |  |  |  |
| Money market instruments                                  | —  | 106,309  | —  | 106,309      |  |  |  |  |
| Total cash and cash equivalents                           | 290,731  | 106,309  | —  | 397,040      |  |  |  |  |
| <b>Available-for-sale securities:</b>                     |  |  |  |              |  |  |  |  |
| <b>Short-term:</b>  |  |  |  |              |  |  |  |  |
| Certificates of deposit                                   | —  | 56,951   | —  | 56,951       |  |  |  |  |
| Corporate debt securities                                 | —  | 42,673   | —  | 42,673       |  |  |  |  |
| Commercial paper  | —  | 12,733   | —  | 12,733       |  |  |  |  |
| U.S. government agency securities                         | —  | 83,222   | —  | 83,222       |  |  |  |  |
| <b>Long-term:</b>   |  |  |  |              |  |  |  |  |
| Certificates of deposit                                   | —  | 6,969  | —  | 6,969        |  |  |  |  |
| Corporate debt securities                                 | —  | 315,240  | —  | 315,240      |  |  |  |  |
| U.S. government agency securities                         | —  | 103,316  | —  | 103,316      |  |  |  |  |
| Greek government-issued bonds                             | —  | 127  | —  | 127          |  |  |  |  |
| Total available-for-sale securities                       | —  | 621,231  | —  | 621,231      |  |  |  |  |
| <b>Other current assets:</b>                              |  |  |  |              |  |  |  |  |
| Nonqualified Deferred Compensation Plan assets            | —  | 440  | —  | 440          |  |  |  |  |
| Forward foreign currency exchange contract <sup>(1)</sup> | —  | 10,478   | —  | 10,478       |  |  |  |  |
| Restricted investments <sup>(2)</sup>                     | —  | 7,348  | —  | 7,348        |  |  |  |  |
| Total other current assets                                | —  | 18,266   | —  | 18,266       |  |  |  |  |
| <b>Other assets:</b>                                      |  |  |  |              |  |  |  |  |
| Nonqualified Deferred Compensation Plan assets            | —  | 6,362  | —  | 6,362        |  |  |  |  |
| Forward foreign currency exchange contract <sup>(1)</sup> | —  | 3,533  | —  | 3,533        |  |  |  |  |
| Strategic investment <sup>(3)</sup>                       | 18,056   | —  | —  | 18,056       |  |  |  |  |
| Total other assets  | 18,056   | 9,895  | —  | 27,951       |  |  |  |  |
| Total assets  | \$ 308,787   | \$ 755,701   | \$ —   | \$ 1,064,488 |  |  |  |  |
| <b>Liabilities:</b>                                       |  |  |  |              |  |  |  |  |
| <b>Current liabilities:</b>                               |  |  |  |              |  |  |  |  |
| Nonqualified Deferred Compensation Plan liability         | \$ 1,151   | \$ 440   | \$ —   | \$ 1,591     |  |  |  |  |

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|   |                 |                  |                  |                  |
|---|-----------------|------------------|------------------|------------------|
| Forward foreign currency exchange contract <sup>(1)</sup> | —               | 2,008            | —                | 2,008            |
| Contingent acquisition consideration payable              | —               | —                | 52,946           | 52,946           |
| <b>Total current liabilities</b>                          | <b>1,151</b>    | <b>2,448</b>     | <b>52,946</b>    | <b>56,545</b>    |
| Other long-term liabilities:                              |                 |                  |                  |                  |
| Nonqualified Deferred Compensation Plan liability         | 24,341          | 6,362            | —                | 30,703           |
| Forward foreign currency exchange contract <sup>(1)</sup> | —               | 3,057            | —                | 3,057            |
| Contingent acquisition consideration payable              | —               | —                | 32,663           | 32,663           |
| <b>Total other long-term liabilities</b>                  | <b>24,341</b>   | <b>9,419</b>     | <b>32,663</b>    | <b>66,423</b>    |
| <b>Total liabilities</b>                                  | <b>\$25,492</b> | <b>\$ 11,867</b> | <b>\$ 85,609</b> | <b>\$122,968</b> |

(1) See Note 12 to these Condensed Consolidated Financial Statements for further information regarding the derivative instruments.

(2) The restricted investments at September 30, 2016 and December 31, 2015 secure the Company's irrevocable standby letter of credit obtained in connection with certain commercial agreements.

## BIOMARIN PHARMACEUTICAL INC.

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

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

(3)The Company has investments in marketable equity securities measured using quoted prices in an active market that are considered strategic investments. See Note 7 to these Condensed Consolidated Financial Statements for additional discussion regarding the Company's strategic investments.

There were no transfers between levels during the three and nine months ended September 30, 2016.

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 7 to these Condensed Consolidated Financial Statements for further information regarding the Company's financial instruments.

Liabilities measured at fair value using Level 3 inputs consisted 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 Consolidated Statements of Comprehensive 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, 2015      | \$85,609  |
| Addition of contingent acquisition consideration payable related to    |           |
| the purchase of the Merck PKU Business                                 | 138,974   |
| Changes in the fair value of contingent acquisition                    |           |
| consideration payable for continuing development programs              | 7,032     |
| Reduction of fair value related to termination of Kyndrisa             |           |
| development program  | (43,652 ) |
| Reduction of fair value related to termination of reveglucosidase alfa |           |
| development program  | (20,334 ) |

## Foreign exchange remeasurement of Euro denominated contingent

|  |            |
|--|------------|
| acquisition consideration payable                                  | 3,761      |
| Contingent acquisition consideration payable at September 30, 2016 | \$ 171,390 |

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, 2015  | \$4,704 |
| Accretion expense                                  | 79      |
| Reversals  | (250 )  |
| Asset retirement obligations at September 30, 2016 | \$4,533 |

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



## BIOMARIN PHARMACEUTICAL INC.

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

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

## (14) STOCK-BASED COMPENSATION

The Company's stock-based compensation plans include the Amended and Restated 2006 Share Incentive Plan (the Share Incentive Plan) and the ESPP. The Company previously had a 2014 Inducement Plan and a 2012 Inducement Plan, which expired in June 2015 and May 2013, respectively. The Company's stock-based compensation plans are administered by the Compensation Committee of the Company's Board of Directors (the Board), which selects persons to receive awards and determines the number of shares subject to each award and the terms, conditions, performance measures and other provisions of the awards. See Note 18 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015 for additional information related to these stock-based compensation plans.

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

The fair value of each option award is estimated on the date of grant using the Black-Scholes valuation model and the assumptions noted in the tables below. The expected life of options is based on observed historical exercise patterns. Groups of employees that have similar historical exercise patterns were considered separately for valuation purposes, and as of September 30, 2016 the Company has identified two groups with distinctly different exercise patterns. The two groups identified are executive and non-executive employees. The executive employee group has a history of holding options for longer periods than non-executive employees. The expected volatility of stock options is based upon the weighted average of the historical volatility of the Company's common stock. The risk-free interest rate is based on the implied yield on a U.S. Treasury zero-coupon issue with a remaining term equal to the expected term of the option. The dividend yield reflects that the Company has not paid any cash dividends since inception and does not intend to pay any cash dividends in the foreseeable future. The assumptions used to estimate the per share fair value of stock options granted under the 2012 Inducement Plan, the 2014 Inducement Plan and the Share Incentive Plan were as follows:

|                         | Three Months Ended |            | Nine Months Ended |            |
|-------------------------|--------------------|------------|-------------------|------------|
|                         | September 30,      |            | September 30,     |            |
|                         | 2016               | 2015       | 2016              | 2015       |
| Expected volatility     | 38 – 40%           | 41 – 44%   | 36 – 44%          | 39 – 45%   |
| Dividend yield          | 0.0%               | 0.0%       | 0.0%              | 0.0%       |
| Expected life           | 5.0 – 6.7 years    |            | 6.4 – 8.0 years   |            |
| Risk-free interest rate | 1.1 – 1.4%         | 1.9 – 2.2% | 5.0 – 8.1 years   | 1.5 – 2.2% |

During the nine months ended September 30, 2016, the Company granted options to purchase 783,580 shares of common stock with a weighted average fair value of \$41.15 per share.

The Company did not issue any new stock purchase rights under the ESPP during the three months ended September 30, 2016.

#### 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 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, 2016, the Company granted 1,223,654 RSUs with a weighted average fair value of \$83.81 per share.

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

During 2012 and 2011, pursuant to the approval of the Board, the Company granted 860,000 RSU awards with performance and market-based vesting conditions (the 2011/2012 Base RSUs) under the Share Incentive Plan and the 2012 Inducement Plan to certain executive officers. The 2011/2012 Base RSUs had a weighted-average grant date fair value of \$34.66 and vested on February 29, 2016, based upon the achievement of the Vimizim approval and the 2015 revenue goal. The Company determined the number of Base RSUs that were earned on the vesting date (the Earned RSUs) and to determine the total number of RSUs to be awarded the Earned RSUs were multiplied by the Total Shareholder Return multiplier of 124% resulting in the issuance of 799,800 shares on February 29, 2016. Stock-based compensation expense for this award was recognized over the service period beginning in the period the Company determined the achievement of the strategic performance goal or goals were probable.

## 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 Conditions

On March 15, 2016, pursuant to Board approval, the Company granted 130,310 RSU awards with performance-vesting conditions (the 2016 Base RSUs) under the Share Incentive Plan to certain executive officers. The vesting of the 2016 Base RSUs under this specific grant is contingent upon the achievement of a 2016 revenue target and a three-year service period. The number of RSUs to be awarded from the 2016 Base RSUs upon achievement of the performance condition shall be calculated by multiplying the 2016 Base RSUs by a revenue multiplier (determined based on the Company's performance against the revenue target), which could range between 80% and 120%.

Stock-based compensation for these awards will be recognized over the service period beginning in the period the Company determines it is probable that the revenue target will be achieved. The cost of the 2016 Base RSUs was determined to be \$83.43 per RSU, based on the fair value of the common stock underlying the 2016 Base RSUs on the grant date. The Company evaluated the 2016 revenue target in the context of its current 2016 revenue forecast and related confidence level in the forecast, and determined that attainment of the revenue target was probable for accounting purposes in the first quarter of 2016. As a result, the Company recognized \$1.0 million and \$2.1 million of compensation expense related to these awards during the three and nine months ended September 30, 2016, respectively.

On March 3, 2015, pursuant to Board approval, the Company granted 58,300 RSU awards with performance-vesting conditions (the 2015 Base RSUs) and a three-year service period, under the Share Incentive Plan, to certain executive officers. Based on the Company's performance against the 2015 revenue target, the Company applied a multiplier of 111% and issued 64,713 RSU awards with a grant date fair value of \$108.36 per RSU. The Company recognized \$0.6 million and \$1.8 million of compensation expense related to these awards during the three and nine months ended September 30, 2016, respectively. The Company recognized \$0.5 million and \$1.3 million of compensation expense related to these awards during the three and nine months ended September 30, 2015, respectively.

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

|  | Three Months<br>Ended<br>September 30, |          | Nine Months<br>Ended<br>September 30, |          |
|--|--|----------|---------------------------------------|----------|
|  | 2016                                   | 2015     | 2016                                  | 2015     |
| Cost of sales                          | \$2,092                                | \$1,386  | \$5,943                               | \$4,484  |
| R&D                                    | 14,165                                 | 12,578   | 42,929                                | 34,972   |
| SG&A                                   | 16,645                                 | 14,794   | 48,348                                | 41,503   |
| Total stock-based compensation expense | \$32,902                               | \$28,758 | \$97,220                              | \$80,959 |

Stock-based compensation expense of \$9.0 million and \$8.3 million was capitalized into inventory for the nine months ended September 30, 2016 and 2015, respectively. Capitalized stock-based compensation is recognized as Cost of Sales when the related product is sold.

## BIOMARIN PHARMACEUTICAL INC.

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

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

## (15) COMPREHENSIVE LOSS

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income (AOCI) and their effect on the Company's Condensed Consolidated Statements of Comprehensive Loss for the three and nine months ended September 30, 2016 and 2015.

| Details about AOCI Components             | Three Months Ended |         | Nine Months Ended  |          | Consolidated Statement of Comprehensive Loss Classification |
|---|--------------------|---------|--------------------|----------|---|
|   | September 30, 2016 | 2015    | September 30, 2016 | 2015     |   |
| Gains (losses) on cash flow hedges:       |                    |         |                    |          |   |
| Forward foreign currency exchange         |                    |         |                    |          |   |
| contracts                                 | \$1,436            | \$4,411 | \$4,036            | \$13,660 | Net product revenues  |
| Forward foreign currency exchange         |                    |         |                    |          |   |
| contracts                                 | 50                 | 776     | 4,874              | 1,424    | SG&A  |
| Gain (loss) on sale of available-for-sale |                    |         |                    |          |   |
| securities                                | 7                  | 14      | (2,020)            | 3,036    | Other income (expense)                                      |
| Less: Income tax effect of the above      | 2                  | 5       | (735 )             | 1,098    | Provision for (Benefit from) income taxes                   |
|   | \$1,491            | \$5,196 | \$7,625            | \$17,022 | Net loss  |

The following tables summarize changes in the accumulated balances for each component of AOCI, including current period other comprehensive income and reclassifications out of AOCI for the three and nine months ended September 30, 2016 and 2015.

|  | Three Months Ended September 30, 2016         |  |                        |         |
|--|---|--|------------------------|---------|
|  | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) on Available-for-Sale Securities | Foreign Currency Items | Total   |
| AOCI balance at June 30, 2016            | \$2,305                                       | \$ 2,231   | \$ (8 )                | \$4,528 |
| Other comprehensive income (loss) before | (1,984)                                       | 1,738  | (1 )                   | (247 )  |

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|  |           |          |        |           |
|--|-----------|----------|--------|-----------|
| reclassifications                                    |           |          |        |           |
| Less net gain reclassified from AOCI                 | 1,486     | 7        | —      | 1,493     |
| Tax effect   | —         | (630)    | )      | (630)     |
| Net current-period other comprehensive income (loss) | (3,470)   | 1,101    | (1)    | (2,370)   |
| AOCI balance at September 30, 2016                   | \$(1,165) | \$ 3,332 | \$ (9) | ) \$2,158 |

Nine Months Ended September 30, 2016

Unrealized

Gains

(Losses) Unrealized Gains  
on Cash (Losses) on Foreign  
Flow Available-for-Sale Currency  
Hedges Securities Items Total

|                                   |          |          |         |            |
|-----------------------------------|----------|----------|---------|------------|
| AOCI balance at December 31, 2015 | \$13,602 | \$ 7,441 | \$ (10) | ) \$21,033 |
|-----------------------------------|----------|----------|---------|------------|

Other comprehensive income (loss) before

|  |           |            |        |           |
|--|-----------|------------|--------|-----------|
| reclassifications                                    | (5,857)   | ) (8,477)  | ) 1    | (14,333)  |
| Less net gain (loss) reclassified from AOCI          | 8,910     | (2,020)    | )      | — 6,890   |
| Tax effect   | —         | 2,348      | —      | 2,348     |
| Net current-period other comprehensive income (loss) | (14,767)  | (4,109)    | ) 1    | (18,875)  |
| AOCI balance at September 30, 2016                   | \$(1,165) | ) \$ 3,332 | \$ (9) | ) \$2,158 |

## BIOMARIN PHARMACEUTICAL INC.

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

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

|  | Three Months Ended September 30, 2015 |  |                        |           |
|--|---------------------------------------|--|------------------------|-----------|
|  | Unrealized Gains                      |  |                        |           |
|  | (Losses) on Cash Flow Hedges          | Unrealized Gains (Losses) on Available-for-Sale Securities | Foreign Currency Items | Total     |
| AOCI balance at June 30, 2015                              | \$ 17,074                             | \$ 15,336  | \$ 51                  | \$ 32,461 |
| Other comprehensive income (loss) before reclassifications | 3,126                                 | (8,159)  | 1                      | (5,032)   |
| Less gain reclassified from AOCI                           | 5,187                                 | 14   | —                      | 5,201     |
| Tax effect   | —                                     | 2,956  | —                      | 2,956     |
| Net current-period other comprehensive loss                | (2,061)                               | (5,217)  | 1                      | (7,277)   |
| AOCI balance at September 30, 2015                         | \$ 15,013                             | \$ 10,119  | \$ 52                  | \$ 25,184 |

|  | Nine Months Ended September 30, 2015 |  |                        |           |
|--|--------------------------------------|--|------------------------|-----------|
|  | Unrealized Gains                     |  |                        |           |
|  | (Losses) on Cash Flow Hedges         | Unrealized Gains (Losses) on Available-for-Sale Securities | Foreign Currency Items | Total     |
| AOCI balance at December 31, 2014                    | \$ 15,906                            | \$ 11,511  | \$ 49                  | \$ 27,466 |
| Other comprehensive income before reclassifications  | 14,191                               | 865  | 3                      | 15,059    |
| Less gain reclassified from AOCI                     | 15,084                               | 3,036  | —                      | 18,120    |
| Tax effect   | —                                    | 779  | —                      | 779       |
| Net current-period other comprehensive income (loss) | (893)                                | (1,392)  | 3                      | (2,282)   |
| AOCI balance at September 30, 2015                   | \$ 15,013                            | \$ 10,119  | \$ 52                  | \$ 25,184 |

## (16) REVENUE AND CREDIT CONCENTRATIONS

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

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

|   | Three Months<br>Ended<br>September 30,<br>2016 |   | 2015 |   | Nine Months<br>Ended<br>September 30,<br>2016 |   | 2015 |   |
|---|--|---|------|---|---|---|------|---|
| Net product revenues marketed by the Company        |  |   |      |   |   |   |      |   |
| United States                                       | 38   | % | 44   | % | 38  | % | 38   | % |
| Europe  | 23   | % | 21   | % | 23  | % | 19   | % |
| Latin America                                       | 14   | % | 12   | % | 13  | % | 19   | % |
| Rest of world                                       | 16   | % | 13   | % | 19  | % | 15   | % |
| Total net product revenue marketed by the Company   | 91   | % | 90   | % | 93  | % | 91   | % |
| Aldurazyme net product revenues marketed by Genzyme | 9  | % | 10   | % | 7   | % | 9    | % |
| Total net product revenues                          | 100  | % | 100  | % | 100   | % | 100  | % |



## 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 illustrates the percentage of the Company's consolidated net product revenues attributed to the Company's largest customers for the three and nine months ended September 30, 2016 and 2015.

|            | Three Months<br>Ended<br>September 30, |      | Nine Months<br>Ended<br>September 30, |      |
|------------|--|------|---------------------------------------|------|
|            | 2016                                   | 2015 | 2016                                  | 2015 |
| Customer A | 19 %                                   | 15 % | 19 %                                  | 14 % |
| Customer B | 13 %                                   | 9 %  | 13 %                                  | 11 % |
| Customer C | 6 %                                    | 6 %  | 6 %                                   | 13 % |
| Customer D | 10 %                                   | 13 % | 10 %                                  | 5 %  |
| Total      | 48 %                                   | 43 % | 48 %                                  | 43 % |

On a consolidated basis, the Company's two largest customers accounted for 22% and 19% of the September 30, 2016 accounts receivable balance, respectively, compared to December 31, 2015, when the two largest customers accounted for 37% and 18% of the accounts receivable balance, respectively. Genzyme is the Company's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. As of September 30, 2016, and December 31, 2015, the accounts receivable balance for Genzyme included \$22.6 million and \$36.1 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.

The Company is subject to credit risk from accounts receivable related to product sales. The majority of the Company's trade accounts receivable arises from product sales in the U.S. and the EU. The Company's product sales to government-owned or government-funded customers in certain European countries, including Italy, Spain, Portugal, Greece and Russia, are subject to payment terms that are statutorily determined. Because these customers are government-owned or government-funded, the Company may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings or a default in these countries may decrease the likelihood that the Company will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to the Company's operating results. In the three and nine months ended September 30, 2016, the Company's net product revenues for these countries were 8% and 7%, respectively. Additionally, approximately 12% of the Company's outstanding accounts receivable at September 30, 2016 related to such countries.

As of September 30, 2016, the Company's accounts receivable in certain European countries, specifically Italy, Spain, Portugal, Greece and Russia, totaled approximately \$26.4 million, of which \$2.6 million were greater than 90 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.

## 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) SEGMENT INFORMATION

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

|                                  | Three Months Ended<br>September 30, |           | Nine Months Ended<br>September 30, |           |
|----------------------------------|-------------------------------------|-----------|------------------------------------|-----------|
|                                  | 2016                                | 2015      | 2016                               | 2015      |
| Net product revenues by product: |                                     |           |                                    |           |
| Vimizim                          | \$80,903                            | 65,106    | \$260,310                          | \$169,608 |
| Naglazyme                        | 77,728                              | 54,131    | 221,575                            | 243,386   |
| Kuvan                            | 90,899                              | 64,219    | 257,806                            | 174,501   |
| Aldurazyme                       | 23,751                              | 20,509    | 58,819                             | 58,991    |
| Firdapse                         | 4,981                               | 3,802     | 13,685                             | 11,616    |
| Total net product revenues       | \$278,262                           | \$207,767 | \$812,195                          | \$658,102 |

The following table summarizes total revenues from external customers and collaborative partners by geographic region. Net product revenues by geographic region are based on patient location for Vimizim, Naglazyme, Kuvan and Firdapse and Genzyme's headquarters for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties earned by the Company on Genzyme's net sales are included in the U.S. region, as the transactions are with Genzyme whose headquarters are located in the U.S.

|                                      | Three Months Ended<br>September 30, |           | Nine Months Ended<br>September 30, |           |
|--------------------------------------|-------------------------------------|-----------|------------------------------------|-----------|
|                                      | 2016                                | 2015      | 2016                               | 2015      |
| Total revenues by geographic region: |                                     |           |                                    |           |
| United States                        | \$130,356                           | \$112,254 | \$365,674                          | 310,209   |
| Europe                               | 62,821                              | 42,816    | 187,328                            | 124,252   |
| Latin America                        | 38,789                              | 25,588    | 106,803                            | 127,147   |
| Rest of world                        | 47,930                              | 28,246    | 156,958                            | 100,351   |
| Total revenues                       | \$279,896                           | \$208,904 | \$816,763                          | \$661,959 |

(18) COMMITMENTS AND CONTINGENCIES

Contingencies

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

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

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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)

Paragraph IV Notices

The Company received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying it that Par had filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of the Company's patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Together with Merck & Cie, on March 6, 2015, the Company filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of its patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe its patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

The Company also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying the Company that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of the Company's patents listed in the FDA's Orange Book. On February 22, 2016, the Company filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of its patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe its patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

The two cases against Par have been consolidated in the District of New Jersey. The court held a claim construction hearing on May 5, 2016 but has not yet issued its ruling. Fact discovery closed in September 2016 and trial is scheduled for June 2017.

SEC Subpoena

In August 2016, the Company received a subpoena from the staff of the SEC requesting that the Company produce documents in connection with a non-public, fact-finding inquiry related to its former drisapersen program. The letter enclosing the subpoena states that the investigation and the subpoena do not mean that the Company or anyone else has broken the law, or that the SEC has a negative opinion of any person, entity or security. The Company intends to cooperate fully with the SEC in this matter. The Company is not able to predict whether any proceeding may be instituted in connection with the subpoena, or the outcome of any proceeding that may be instituted.

Contingent Payments

As of September 30, 2016, the Company is subject to contingent payments totaling approximately \$754.4 million upon achievement of certain development and regulatory activities and commercial sales and licensing milestones if they occur before certain dates in the future. Of this amount, \$207.6 million (or €185 million based on the exchange rate of 1.12 USD per Euro in effect on September 30, 2016) relates to the Merck PKU Business acquisition and \$58.2 million relates to programs that are no longer being developed.

As of September 30, 2016, the Company has recorded \$171.4 million of contingent acquisition consideration payable on its Condensed Consolidated Balances Sheets in Short-term and Long-term Contingent Acquisition Consideration Payable, of which \$48.7 million is expected to be paid in the next twelve months.

#### Other Commitments

In the normal course of business, the Company enters into various firm purchase commitments primarily related to active pharmaceutical ingredients and certain inventory related items. As of September 30, 2016, these commitments for the next five years were approximately \$50.9 million. The amounts primarily represent minimum purchase requirements for active pharmaceutical ingredients and post-marketing commitments related to the Company's approved products.

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) BENEFIT FROM INCOME TAXES

The Company has historically computed its interim period benefit from income taxes by applying its forecasted effective tax rate to year-to-date earnings. However, due to a significant amount of U.S. permanent differences relative to the amount of U.S. forecasted income used in computing the effective tax rate, the effective tax rate can be highly sensitive to minor fluctuations in U.S. forecasted income. As such, the Company has computed the U.S. component of the consolidated benefit from income taxes for the three and nine months ended September 30, 2016 using an actual year-to-date tax calculation. Foreign tax expense was computed using a forecasted annual effective tax rate for the three and nine months ended September 30, 2016. The consolidated benefit from income taxes for the three and nine months ended September 30, 2015 was computed using a forecasted annual effective tax rate.

## 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," "intends," "anticipates," "plans," "may," "will," "projects," "continues," "estimates," "potential," "opportunity" or the negative versions of these terms and other similar expressions. These forward-looking statements may be found in the part of this Item 2 entitled "Overview" 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, 2015, which was filed with the Securities and Exchange Commission (the SEC) on February 29, 2016. You should carefully consider that information before you make an investment decision.

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

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

### Overview

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

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

### Business Developments

During 2016, we continued to grow our commercial business and advance our product pipeline. We believe that the combination of our internal research programs, acquisitions and partnerships will allow us to continue develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. Below is a summary of key business developments in 2016 to date:

In October 2016, we provided an update on our Phase 2 study of vosoritide, an analog of C-type Natriuretic Peptide, in children with achondroplasia, the most common form of dwarfism. Results from eight children in cohort 4, who completed six months of daily dosing at 30  $\mu\text{g}/\text{kg}/\text{daily}$ , experienced a 46% or 2.1 cm/year increase in mean annualized growth velocity from baseline. These data are comparable to those observed at the lower dose of 15  $\mu\text{g}/\text{kg}/\text{day}$  in cohort 3. Results from 10 children in cohort 3, who completed six months of daily dosing at 15  $\mu\text{g}/\text{kg}/\text{day}$ , experienced a 50% or 2.0 cm/year increase in mean annualized growth velocity from baseline.



In September 2016, we announced that the European Medicines Agency (EMA) validated the Marketing Authorization Application (MAA) for Brineura (formerly referred to as cerliponase alfa), an investigational therapy to treat children with CLN2 disease, a form of batten disease. Validation of the MAA confirmed that the submission was accepted and starts the formal review process by the EMA's Committee for Human Medicinal Products (CHMP). The EMA previously granted our request for accelerated assessment for the MAA. The CHMP opinion and decision from the European Commission (EC) is expected in the third quarter of 2017. Accelerated assessments are granted on the grounds that a product may satisfy an unmet medical need and is of major interest from the point of view of therapeutic innovation and public health.

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In July 2016, we announced that the United States Food and Drug Administration (FDA) accepted for review the submission of a Biologics License Application (BLA) for Brineura. During their initial review of the BLA, the FDA requested updated efficacy data from the ongoing extension study, which we provided. In September 2016, the FDA designated this submission as a major amendment to the application, thus extending the Prescription Drug User Fee Act (PDUFA) action date by three months to April 27, 2017. The FDA granted Brineura Priority Review status, which is designated to drugs that, if approved, would be a significant improvement in treatment or provide a treatment where no adequate therapy exists. Brineura was previously granted Orphan Drug Designation by the FDA and EMA and Breakthrough Therapy Designation by the FDA.

In July 2016, we announced positive interim results of an open-label, Phase 1/2 study of BMN 270, an investigational gene therapy treatment for severe hemophilia A at the XXXII International Congress of the World Federation of Hemophilia. Earlier in the year, we received Orphan Drug Designation from the FDA for BMN 270 for hemophilia A.

In June 2016, we announced that the reveglucosidase alfa development program has been terminated and that a partner is being sought to assume future studies. We recognized an impairment charge of \$25.0 million in the second quarter of 2016 related to the reveglucosidase alfa in-process research and development (IPR&D) assets.

In May 2016, we withdrew our MAA from the EMA for Kyndrisa (drisapersen). We discontinued clinical and regulatory development of Kyndrisa as well as the three other first generation follow-on products, BMN 044, BMN 045 and BMN 053 (other exons). We recognized an impairment charge of \$574.1 million in the second quarter of 2016 related to the Kyndrisa and other exon IPR&D assets.

In April 2016, we announced enrollment of the first patient in a Phase 1/2 trial for NAGLU (referred to as BMN 250), an investigational enzyme replacement therapy using a novel fusion of recombinant human alpha-N-acetylglucosaminidase with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or mucopolysaccharidosis IIIB (MPS IIIB).

In March 2016, we announced that our pivotal Phase 3 PRISM-2 study of pegvaliase met the primary endpoint of change in blood Phe compared with placebo ( $p < 0.0001$ ). Based on the supportive data results, the Company plans to submit a BLA to the FDA in the first quarter of 2017.

In January 2016, we acquired all global rights to Kuvan and pegvaliase, with the exception of Kuvan in Japan, (collectively, the Merck PKU Business) from Ares Trading S.A. (Merck Serono), an indirectly wholly-owned affiliate of Merck KGaA, in exchange for cash payments of \$374.5 million in the nine months ended September 30, 2016. We also agreed to pay Merck Serono up to a maximum of €60.0 million in milestones if certain sales milestones are met and up to a maximum of €125.0 million if certain pegvaliase development milestones are met. See Note 5 to our accompanying Condensed Consolidated Financial Statements for additional discussion.

We reported total revenues of \$279.9 million and \$816.8 million for the three and nine months ended September 30, 2016 as compared to \$208.9 million and \$662.0 million for the three and nine months ended September 30, 2015.

#### Financial Highlights

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

|  | Three Months<br>Ended<br>September 30, |         | Nine Months<br>Ended<br>September 30, |         |
|--|--|---------|---------------------------------------|---------|
|  | 2016                                   | 2015    | 2016                                  | 2015    |
| Total revenues                                     | \$279.9                                | \$208.9 | \$816.8                               | \$662.0 |
| Cost of sales                                      | 50.7                                   | 34.9    | 145.5                                 | 104.0   |
| Research and Development (R&D) expense             | 160.8                                  | 158.7   | 486.7                                 | 458.7   |
| Selling, general and administrative (SG&A) expense | 118.8                                  | 94.0    | 333.6                                 | 288.4   |
| Intangible asset amortization and                  | 9.7                                    | 3.1     | (34.3 )                               | 23.0    |

|                                  |        |        |         |         |
|----------------------------------|--------|--------|---------|---------|
| contingent consideration expense |        |        |         |         |
| Impairment of intangible assets  | —      | —      | 599.1   | —       |
| Net loss                         | (42.8) | (90.9) | (551.5) | (240.4) |
| Stock-based compensation expense | 32.9   | 28.8   | 97.2    | 81.0    |

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

Net product revenues are generated from the five approved products in our product portfolio. In the U.S., our commercial products are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. Outside the U.S., our commercial products are sold to our authorized distributors or directly to government purchasers or hospitals, which act as the end-users. Collaborative agreement revenues include both license revenue and contract research revenue. Royalty, license and other revenues include royalties on net sales of products to licensees or sublicensees and rental income associated with the tenants in our San Rafael, California facility.

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

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

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

Our cash, cash equivalents, short-term investments and long-term investments totaled \$1.4 billion as of September 30, 2016, compared to \$1.0 billion as of December 31, 2015. We have historically financed our operations primarily through our cash flows from operating activities and the issuance of common stock and convertible debt. We will be highly dependent on our net product revenues 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 United States (U.S.) and pursuant to the rules and regulations promulgated by the SEC, we make assumptions, judgments and estimates that can have a significant impact on our net income/loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. We base our assumptions, judgments and estimates on 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.

There have been no significant changes to our critical accounting policies and estimates during the nine months ended September 30, 2016, 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, 2015.

#### Recent Accounting Pronouncements

See Note 4 to our accompanying Condensed Consolidated Financial Statements for a 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 Loss

Our net loss for the three months ended September 30, 2016, was \$42.8 million, compared to a net loss of \$90.9 million for the three months ended September 30, 2015. Our net loss for the nine months ended September 30, 2016 was \$551.5 million, compared to a net loss of \$240.4 million for the nine months ended September 30, 2015. The increase in net loss was primarily a result of the following (in millions):

|  | Three Months Ended |           |         | Nine Months Ended  |           |           |
|--|--------------------|-----------|---------|--------------------|-----------|-----------|
|  | September 30, 2016 | 2015      | Change  | September 30, 2016 | 2015      | Change    |
| Total revenues   | \$279.9            | \$208.9   | \$71.0  | \$816.8            | \$662.0   | \$154.8   |
| Cost of sales  | 50.7               | 34.9      | 15.8    | 145.5              | 104.0     | 41.5      |
| R&D expense  | 160.8              | 158.7     | 2.1     | 486.7              | 458.7     | 28.0      |
| SG&A expense   | 118.8              | 94.0      | 24.8    | 333.6              | 288.4     | 45.2      |
| Intangible asset amortization and contingent consideration | 9.7                | 3.1       | 6.6     | (34.3 )            | 23.0      | (57.3 )   |
| Impairment of intangible assets                            | —                  | —         | —       | 599.1              | —         | 599.1     |
| Other, net   | (6.7 )             | (8.6 )    | 1.9     | (25.1 )            | (35.6 )   | 10.5      |
| Benefit from income taxes                                  | (24.0 )            | 0.5       | (24.5 ) | (187.4)            | (7.3 )    | (180.1)   |
| Net loss   | \$(42.8 )          | \$(90.9 ) | \$48.1  | \$(551.5)          | \$(240.4) | \$(311.1) |

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

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

A summary of our various commercial products, including key metrics as of September 30, 2016, is provided below:

| Commercial Products       | Indication             | Orphan Drug Exclusivity Expiration U.S. | Orphan Drug Exclusivity Expiration EU |
|---------------------------|------------------------|---|---------------------------------------|
| Vimizim                   | MPS IVA <sup>(1)</sup> | 2021                                    | 2024                                  |
| Naglazyme                 | MPS VI <sup>(2)</sup>  | Expired                                 | Expired                               |
| Kuvan                     | PKU <sup>(3)</sup>     | Expired                                 | 2020 <sup>(4)</sup>                   |
| Aldurazyme <sup>(5)</sup> | MPS I <sup>(6)</sup>   | Expired                                 | Expired                               |
| Firdapse                  | LEMS <sup>(7)</sup>    | NA <sup>(8)</sup>                       | 2019                                  |

(1)Mucopolysaccharidosis IV Type A (MPS IVA).

(2)Mucopolysaccharidosis VI (MPS VI).

(3)Phenylketonuria (PKU).

(4)Kuvan has been granted orphan drug status in the EU that, together with pediatric exclusivity, confers 12 years of market exclusivity in the EU that expires in 2020. Merck Serono marketed Kuvan in the EU until January 1, 2016.

(5)The Aldurazyme net product revenues noted above are the net product revenues recognized by us in accordance with the terms of our agreement with Genzyme Corporation (Genzyme).

(6)Mucopolysaccharidosis I (MPS I).

(7)Lambert Eaton Myasthenic Syndrome (LEMS).

(8) Firdapse has not received marketing approval in the U.S. and we have licensed the North American rights to develop and market Firdapse to Catalyst Pharmaceutical Partners, Inc.

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

|                            | Three Months Ended<br>September 30, |         |         | Nine Months Ended<br>September 30, |         |         |
|----------------------------|-------------------------------------|---------|---------|------------------------------------|---------|---------|
|                            | 2016                                | 2015    | Change  | 2016                               | 2015    | Change  |
| Vimizim                    | \$80.9                              | \$65.1  | \$ 15.8 | \$260.3                            | \$169.6 | \$90.7  |
| Naglazyme                  | 77.8                                | 54.1    | 23.7    | 221.6                              | 243.4   | (21.8 ) |
| Kuvan                      | 90.9                                | 64.2    | 26.7    | 257.8                              | 174.5   | 83.3    |
| Aldurazyme                 | 23.7                                | 20.6    | 3.1     | 58.8                               | 59.0    | (0.2 )  |
| Firdapse                   | 5.0                                 | 3.8     | 1.2     | 13.7                               | 11.6    | 2.1     |
| Total net product revenues | \$278.3                             | \$207.8 | \$ 70.5 | \$812.2                            | \$658.1 | \$154.1 |

Our Vimizim, Naglazyme, Kuvan and Firdapse customers include a limited number of specialty pharmacies and end users, such as hospitals and foreign government agencies. We also sell Vimizim and Naglazyme to our authorized distributors and to certain larger pharmaceutical wholesalers globally, which act as intermediaries between us and end users and generally do not stock significant quantities of our products. However, in certain countries, particularly in Latin America, governments place large periodic orders for Vimizim and Naglazyme. The timing of these large government orders can be inconsistent and can create significant quarter to quarter variation in our revenues. During each of the three and nine months ended September 30, 2016, 42% of our net product revenues were generated by three customers. Genzyme is our sole customer for Aldurazyme and is responsible for the global marketing and selling of Aldurazyme to third-parties.

Vimizim – The FDA and the EMA granted marketing approval for Vimizim in February 2014 and April 2014, respectively, and Vimizim subsequently received marketing approval in other countries. We began marketing Vimizim immediately following approval in each of these markets. The following table shows our net product revenues for Vimizim:

|   | Three Months Ended<br>September 30, |        |         | Nine Months Ended<br>September 30, |         |         |
|---|-------------------------------------|--------|---------|------------------------------------|---------|---------|
|   | 2016                                | 2015   | Change  | 2016                               | 2015    | Change  |
| Sales denominated in U.S. Dollars (USD) | \$40.6                              | \$29.5 | \$ 11.1 | \$125.7                            | \$88.1  | \$ 37.6 |
| Sales denominated in foreign currencies | 40.3                                | 35.6   | 4.7     | 134.6                              | 81.5    | 53.1    |
| Total Vimizim net product revenues      | \$80.9                              | \$65.1 | \$ 15.8 | \$260.3                            | \$169.6 | \$ 90.7 |

The increase in Vimizim net product revenues for the three and nine months ended September 30, 2016, compared to the three and nine months ended September 30, 2015, was primarily attributable to new patients initiating therapy and in part to the timing of orders from Latin America and the Middle East. The impact of foreign currency exchange rates on Vimizim sales denominated in currencies other than USD was positive by \$0.5 million and negative by \$0.9 million for the three and nine months ended September 30, 2016, respectively. The impact of foreign currency exchange rates on Vimizim sales denominated in currencies other than USD was negative by \$6.1 million and \$14.3 million for the three and nine months ended September 30, 2015, respectively. Gross margin (net product revenues less costs of sales, expressed as a percentage of net product revenues) for Vimizim was 83% and 84% for each of the three and nine months ended September 30, 2016, respectively, compared to gross margin of 84% for each of the three and nine months ended September 30, 2015, respectively. In future periods, we do not expect Vimizim gross margins to fluctuate significantly.

Naglazyme – The following table shows our net product revenues for Naglazyme:



|   | Three Months Ended |        |         | Nine Months Ended |         |           |
|---|--------------------|--------|---------|-------------------|---------|-----------|
|   | September 30,      |        |         | September 30,     |         |           |
|   | 2016               | 2015   | Change  | 2016              | 2015    | Change    |
| Sales denominated in USD                | \$35.1             | \$16.2 | \$ 18.9 | \$94.4            | \$117.1 | \$(22.7 ) |
| Sales denominated in foreign currencies | 42.7               | 37.9   | 4.8     | 127.2             | 126.3   | 0.9       |
| Total Naglazyme net product revenues    | \$77.8             | \$54.1 | \$ 23.7 | \$221.6           | \$243.4 | \$(21.8 ) |

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The increase in Naglazyme net product revenues for the three months ended September 30, 2016, compared to the three months ended September 30, 2015, was primarily attributable to the timing of central government orders from Latin America and new patients initiating therapy. The decrease in Naglazyme net product revenues for the nine months ended September 30, 2016, compared to the nine months ended September 30, 2015, was attributable to the timing of central government orders from Latin America, offset in part by new patients initiating therapy. The impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than USD was immaterial for the three months ended September 30, 2016, and negative by \$1.5 million for the nine months ended September 30, 2016. The impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than USD was negative by \$2.2 million and \$10.5 million for the three and nine months ended September 30, 2015, respectively. Our gross margin for Naglazyme was 84% and 85% for the three and nine months ended September 30, 2016 compared to gross margin of 86% and 87% for the three and nine months ended September 30, 2015, respectively. In future periods, we do not expect Naglazyme gross margins to fluctuate significantly.

Kuvan – The following table shows our net product revenues for Kuvan:

|   | Three Months Ended |        |         | Nine Months Ended  |         |         |
|---|--------------------|--------|---------|--------------------|---------|---------|
|   | September 30, 2016 | 2015   | Change  | September 30, 2016 | 2015    | Change  |
| Sales denominated in USD                | \$68.7             | \$62.3 | \$ 6.4  | \$194.8            | \$168.3 | \$ 26.5 |
| Sales denominated in foreign currencies | 22.2               | 1.9    | 20.3    | 63.0               | 6.2     | 56.8    |
| Total Kuvan net product revenues        | \$90.9             | \$64.2 | \$ 26.7 | \$257.8            | \$174.5 | \$ 83.3 |

The increase in Kuvan net product revenues for the three and nine months ended September 30, 2016, compared to the three and nine months ended September 30, 2015, was primarily attributable to new patients initiating therapy in the U.S. and the addition of international Kuvan product sales through the acquisition of the Merck PKU Business. Our gross margin for Kuvan was 82% and 80% for the three and nine months ended September 30, 2016, respectively, compared to 83% and 84% for the three and nine months ended September 30, 2015, respectively. During the first nine months of 2016, the cost of inventory acquired in the Merck PKU Business acquisition included a fair value adjustment related to the business combination accounting, which reduced gross margin for those units to a reasonable seller's profit. Cost of sales for each of the three and nine months ended September 30, 2016 and 2015 reflect royalties paid to third-parties of up to approximately 10%. We expect the Kuvan inventory purchased from Merck Serono to be depleted during the fourth quarter of 2016 and Kuvan gross margins are expected to normalize as a result, after which we do not expect Kuvan gross margins to fluctuate significantly. Prior to our acquisition of the Merck PKU Business, we earned royalties on Merck Serono's net sales of Kuvan of 4%.

In September 2015, we entered into a settlement agreement with Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (DRL) that resolved patent litigation with DRL in the U.S. related to its abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances. The settlement does not affect the case against Par Pharmaceutical, Inc. (Par) with respect to its 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). Par has also filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder, and the litigation pertaining to Par's purported ANDA for 100 mg oral powder has been consolidated with the case pertaining to Par's purported ANDA for 100 mg oral tablets.

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

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

|  | Three Months Ended<br>September 30, |               |               | Nine Months Ended<br>September 30, |               |                  |
|--|-------------------------------------|---------------|---------------|------------------------------------|---------------|------------------|
|  | 2016                                | 2015          | Change        | 2016                               | 2015          | Change           |
| Aldurazyme revenue reported by Genzyme                 | \$58.9                              | \$53.8        | \$ 5.1        | \$168.5                            | \$163.7       | \$ 4.8           |
| Royalties earned from Genzyme<br>Previously recognized | \$26.9                              | \$23.3        | \$ 3.6        | \$71.2                             | \$69.1        | \$ 2.1           |
| Aldurazyme product transfer revenue                    | (3.2)                               | (2.7)         | (0.5 )        | (12.4)                             | (10.1)        | (2.3 )           |
| <b>Total Aldurazyme net product revenues</b>           | <b>\$23.7</b>                       | <b>\$20.6</b> | <b>\$ 3.1</b> | <b>\$58.8</b>                      | <b>\$59.0</b> | <b>\$ (0.2 )</b> |

The increase in Aldurazyme net product revenues for the three months ended September 30, 2016, compared to the three months ended September 30, 2015, was primarily attributable to an increase in Genzyme-reported Aldurazyme sales. The decrease in Aldurazyme net product revenues for the nine months ended September 30, 2016, compared to the nine months ended September 30, 2015, was primarily attributable to the increase in shipments to Genzyme, offset in part by the increase in Aldurazyme revenue reported by Genzyme. Our gross margin on Aldurazyme was 71% and 75% for the three and nine months ended September 30, 2016, respectively, compared to gross margin of 75% and 77% for the three and nine months ended September 30, 2015, respectively. Although Genzyme sells Aldurazyme worldwide, the royalties earned by the Company on Genzyme’s net sales are denominated in USD. 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 incremental/previously recognized product transfer revenue, from which we earn lower gross profit.

#### Cost of Sales

Total cost of sales for the three and nine months ended September 30, 2016 was \$50.7 million and \$145.5 million, as compared to \$34.9 million and \$104.0 million for the three and nine months ended September 30, 2015. The increase in cost of sales was primarily attributable to the increase in product sales as well as recognition of the fair value adjustment to inventory acquired in the Merck PKU Business acquisition that was sold in the three and nine months ended September 30, 2016.

#### Research and Development

A summary of our on-going major development programs, including key metrics as of September 30, 2016, is provided below:

| Major Products in Development                       | Target Indication   | U.S. Orphan |             | EU Orphan         |                         |
|---|---------------------|-------------|-------------|-------------------|-------------------------|
|   |                     | Designation | Designation | Stage             | Marketing authorization |
| Brineura (formerly referred to as cerliponase alfa) | CLN2 <sup>(1)</sup> | Yes         | Yes         | regulatory review |                         |
| Pegvaliase  | PKU                 | Yes         | Yes         | Clinical Phase 3  |                         |
| Vosoritide  | Achondroplasia      | Yes         | Yes         | Clinical Phase 2  |                         |

|   |                         |     |     |                    |
|---|-------------------------|-----|-----|--------------------|
| BMN 270 <sup>(2)</sup>                  | Hemophilia A            | Yes | Yes | Clinical Phase 1/2 |
| NAGLU (formerly referred to as BMN 250) | MPS IIIB <sup>(3)</sup> | Yes | Yes | Clinical Phase 1/2 |

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

(2) BMN 270 is an investigational gene therapy for Hemophilia A, also called factor VIII deficiency or classic hemophilia.

(3) Sanfilippo B syndrome, or mucopolysaccharidosis type IIIB (MPS IIIB).

We manage our R&D expense by identifying the R&D 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.

R&D expense increased to \$160.8 million and \$486.7 million for the three and nine months ended September 30, 2016, from \$158.7 million and \$458.7 million for the three and nine months ended September 30, 2015. R&D expense consisted of the following (in millions):

|                                     | Three Months Ended |                |               | Nine Months Ended |                |                |
|-------------------------------------|--------------------|----------------|---------------|-------------------|----------------|----------------|
|                                     | September 30,      |                |               | September 30,     |                |                |
|                                     | 2016               | 2015           | Change        | 2016              | 2015           | Change         |
| Brineura                            | \$20.2             | \$10.0         | \$ 10.2       | \$52.4            | \$29.1         | \$ 23.3        |
| Pegvaliase                          | 19.5               | 18.6           | 0.9           | 59.0              | 52.3           | 6.7            |
| Kyndrisa <sup>(1)</sup>             | 17.7               | 16.2           | 1.5           | 65.2              | 39.2           | 26.0           |
| BMN 270                             | 12.8               | 6.0            | 6.8           | 38.9              | 22.3           | 16.6           |
| NAGLU                               | 11.4               | 9.7            | 1.7           | 35.3              | 25.1           | 10.2           |
| Reveglucosidase alfa <sup>(2)</sup> | 9.1                | 15.2           | (6.1 )        | 37.8              | 45.4           | (7.6 )         |
| Vosoritide                          | 11.6               | 11.2           | 0.4           | 35.5              | 29.8           | 5.7            |
| Vimizim                             | 5.9                | 10.5           | (4.6 )        | 19.7              | 34.7           | (15.0 )        |
| Other approved products             | 10.3               | 10.0           | 0.3           | 30.2              | 26.2           | 4.0            |
| Talazoparib <sup>(3)</sup>          | (0.9 )             | 17.4           | (18.3 )       | 1.8               | 55.4           | (53.6 )        |
| Early stage programs                | 15.3               | 9.6            | 5.7           | 40.0              | 25.8           | 14.2           |
| Other and non-allocated             | 27.9               | 24.3           | 3.6           | 70.9              | 73.4           | (2.5 )         |
| <b>Total</b>                        | <b>\$160.8</b>     | <b>\$158.7</b> | <b>\$ 2.1</b> | <b>\$486.7</b>    | <b>\$458.7</b> | <b>\$ 28.0</b> |

(1)The Kyndrisa and other exon programs were terminated in the first and second quarters of 2016, respectively.

(2)In the second quarter of 2016, we terminated the reveglucosidase alfa development program.

(3)In October 2015, we sold talazoparib to Medivation. For the nine months ended September 30, 2016, talazoparib R&D expense primarily related to changes in estimate and employee-related wind-down costs.

The increase in pegvaliase, Brineura, and vosoritide R&D expense was attributable to increased clinical trial activities related to these product candidates. The development expenses for Kyndrisa relate to clinical and European regulatory activities for this product candidate, which are expected to decrease due to the termination of the related development program, as well as a charge of \$4.9 million for one-time employee termination benefits. The increase in development expense related to early development stage programs was primarily attributable to the pre-clinical activity related to BMN 270 and NAGLU. The decrease in reveglucosidase alfa was due to the termination of the respective development program. The decrease in talazoparib R&D expenses was due to the completion of the sale of the assets to Medivation in the fourth quarter of 2015.

During the remainder of 2016, we expect our R&D spending to increase over 2015 levels due to our pegvaliase, vosoritide and Brineura programs progressing in their development. We also expect increased spending on pre-clinical and clinical activities for our early development stage programs, including BMN 270 and NAGLU, and other pre-clinical programs. Additionally, we expect to continue incurring significant R&D expense for the foreseeable future due to long-term clinical activities related to post-approval regulatory commitments for our approved products. We continuously evaluate the recoverability of costs associated with pre-launch manufacturing activities, and if it is determined that recoverability is highly likely and therefore future revenues are expected, the costs subsequently incurred related to pre-launch manufacturing activities for purposes of commercial sales will likely be capitalized. When regulatory approval and the likelihood of future revenues for a product candidate are less certain, the related manufacturing costs are expensed as R&D expenses. In the second quarter of 2016, we began capitalizing Brineura pre-launch manufacturing costs incurred in preparation for anticipated commercial sales.

## Selling, General and Administrative

SG&A expense increased to \$118.8 million and \$333.6 million for the three and nine months ended September 30, 2016, respectively, from \$94.0 million and \$288.4 million for the three and nine months ended September 30, 2015, respectively. The increase in SG&A expense was primarily a result of the following (in millions):

|  | Three Months Ended |        |         | Nine Months Ended |         |         |
|--|--------------------|--------|---------|-------------------|---------|---------|
|  | September 30,      |        |         | September 30,     |         |         |
|  | 2016               | 2015   | Change  | 2016              | 2015    | Change  |
| Sales and marketing (S&M) expense        | \$61.3             | \$49.2 | \$ 12.1 | \$174.9           | \$142.0 | \$ 32.9 |
| General and administrative (G&A) expense | 57.5               | 44.8   | 12.7    | 158.7             | 146.4   | 12.3    |
| Total SG&A expense                       | \$118.8            | \$94.0 | \$ 24.8 | \$333.6           | \$288.4 | \$ 45.2 |

| S&M expense by product  | Three Months Ended<br>September 30, |        |         | Nine Months Ended<br>September 30, |         |         |
|-------------------------|-------------------------------------|--------|---------|------------------------------------|---------|---------|
|                         | 2016                                | 2015   | Change  | 2016                               | 2015    | Change  |
| Vimizim                 | \$16.9                              | \$12.9 | \$ 4.0  | \$48.0                             | \$40.5  | \$ 7.5  |
| Naglazyme               | 12.6                                | 10.7   | 1.9     | 36.6                               | 34.2    | 2.4     |
| Kuvan                   | 13.6                                | 9.9    | 3.7     | 42.9                               | 30.1    | 12.8    |
| Other and not allocated | 18.2                                | 15.7   | 2.5     | 47.4                               | 37.2    | 10.2    |
| Total S&M expense       | \$61.3                              | \$49.2 | \$ 12.1 | \$174.9                            | \$142.0 | \$ 32.9 |

S&M expense primarily consists of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. The increase in Kuvan S&M expense is attributable to expansion of worldwide commercial activities as a result of acquiring the worldwide rights to Kuvan, except for Japan, on January 1, 2016. We continue to incur S&M expense for Naglazyme and Vimizim as a result of continued expansion of our worldwide commercial activities. The increase in other S&M expense was driven by an increase in pre-commercialization marketing expense for Brineura.

G&A expense primarily consists of corporate support and other administrative expenses, which increased in the three and nine months ended September 30, 2016, as compared to the three and nine months ended September 30, 2015, primarily due to increased headcount and the impact of foreign currency fluctuations.

We expect SG&A expense to increase in future periods as a result of pre-commercialization expense related to product candidates, the continued international expansion of Naglazyme, Vimizim and 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 consists of changes in the fair value of contingent acquisition consideration payable in respect of our acquired businesses and amortization of intangible assets. Changes in the fair value of contingent acquisition consideration payable result from updates to the estimated probability of achievement or assumed timing of milestones and adjustments to the discount periods and rates. Intangible asset amortization and contingent consideration expense consisted of the following (in millions):

|   | Three Months Ended<br>September 30, |       |        | Nine Months Ended<br>September 30, |        |           |
|---|-------------------------------------|-------|--------|------------------------------------|--------|-----------|
|   | 2016                                | 2015  | Change | 2016                               | 2015   | Change    |
| Changes in the fair value of contingent |                                     |       |        |                                    |        |           |
| acquisition consideration payable       | \$2.1                               | \$0.5 | \$ 1.6 | \$(57.0)                           | \$15.1 | \$(72.1 ) |
| Amortization of intangible assets       | 7.6                                 | 2.6   | 5.0    | 22.7                               | 7.9    | 14.8      |
| Total intangible asset amortization and |                                     |       |        |                                    |        |           |
| contingent consideration                | \$9.7                               | \$3.1 | \$ 6.6 | \$(34.3)                           | \$23.0 | \$(57.3 ) |

The changes in the fair value of the contingent acquisition consideration payable were primarily attributable to changes in the estimated probability of achieving development milestones based on the current status of the related development programs as well as the passage of time. During the nine months ended September 30, 2016, the majority of the changes related to the discontinuance of the Kyndrisa and reveglucosidase alfa development programs that resulted in the reversal of the fair value of the remaining contingent consideration payable to the former Prosensa and Zystor Therapeutics, Inc. shareholders, because the related sales milestones are no longer expected to be attained. The



increase in amortization of intangible assets during both the three and nine months ended September 30, 2016, was primarily attributable to the amortization of the Kuvan intangible assets acquired from Merck Serono in January 2016.

#### Impairment of Intangible Assets

In the second quarter of 2016, we recorded an impairment charge of \$599.1 million related to the Kyndrisa and other exon and reveglucosidase alfa IPR&D assets based on the termination of the internal development of the respective programs. See Note 8 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our Intangible Assets.

## 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 \$1.6 million and \$4.6 million for the three and nine months ended September 30, 2016, compared to \$1.3 million and \$3.1 million for the three and nine months ended September 30, 2015. Due to low interest rates and planned spend, we do not expect interest income to fluctuate significantly over the next 12 months.

## Interest Expense

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

|  | Three Months Ended<br>September 30, |       |        | Nine Months Ended<br>September 30, |        |           |
|--|-------------------------------------|-------|--------|------------------------------------|--------|-----------|
|  | 2016                                | 2015  | Change | 2016                               | 2015   | Change    |
| Coupon interest                            | \$2.5                               | \$2.3 | \$ 0.2 | \$7.5                              | \$7.7  | \$ (0.2 ) |
| Amortization of issuance costs             | 0.8                                 | 0.8   | —      | 2.5                                | 2.5    | —         |
| Accretion of discount on convertible notes | 6.7                                 | 6.3   | 0.4    | 19.8                               | 18.7   | 1.1       |
| Total interest expense                     | \$10.0                              | \$9.4 | \$ 0.6 | \$29.8                             | \$28.9 | \$ 0.9    |

Interest expense primarily consisted of amounts related to our October 2013 issuance of \$750.0 million in aggregate principal amount of senior subordinated convertible debt. We do not expect future interest expense to fluctuate significantly over the next twelve months. See Note 11 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our Convertible Debt.

## Other Expense

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

## Provision for (Benefit from) Income Taxes

For the three and nine months ended September 30, 2016, we recognized a benefit from income taxes of \$24.0 million and \$187.4 million, respectively, compared to the three and nine months ended September 30, 2015 when we recognized income tax expense of \$0.5 million and an income tax benefit of \$7.3 million, respectively. The benefit from income taxes for the nine months ended September 30, 2016 primarily included the reversal of the deferred tax liability associated with the write-off of the IPR&D related to Kyndrisa and reveglucosidase alfa. We have historically computed our interim period provision for (benefit from) income taxes by applying our forecasted effective tax rate to year-to-date earnings. However, due to a significant amount of U.S. permanent differences relative to the amount of U.S. forecasted income used in computing the effective tax rate, the effective tax rate can be highly sensitive to minor fluctuations in U.S. forecasted income. As such, we have computed the U.S. component of the consolidated benefit from income taxes for the three and nine months ended September 30, 2016 using an actual year-to-date tax calculation. Foreign tax expense was computed using a forecasted annual effective tax rate for the three and nine months ended September 30, 2016. The consolidated provision for (benefit from) income taxes for the three and nine months ended September 30, 2015 was computed using a forecasted annual effective tax rate.

The provision for (benefit from) income taxes for the three and nine months ended September 30, 2016 and 2015 also consisted of state, federal and foreign current tax expense that was offset by deferred tax benefits from federal orphan

drug and the federal and California R&D credits, and the tax benefit related to stock option exercises during these periods. See Note 16 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2015 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, and proceeds from equity or debt financings and loans or collaborative agreements with corporate partners. We will be highly dependent on our net product revenues to supplement our current liquidity and fund our operations for the foreseeable future. We plan to supplement this with further debt or equity offerings or commercial borrowing.

We consider the unrepatriated cumulative earnings of certain of our foreign subsidiaries to be indefinitely invested outside the U.S. As of September 30, 2016, \$119.0 million of our \$1.4 billion balance of cash, cash equivalents, short-term and long-term investments was held in foreign subsidiaries, a significant portion of which is required to fund the liquidity needs of these foreign subsidiaries. For additional discussion, see Note 16 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2015.

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. or other countries, a continuation of uncertainty with respect to, or worsening of global economic conditions, patent expirations of competitive products and the launch of generic competitors, continued government pricing pressures internationally and the potential volatility in foreign currency exchange rates. We will continue to monitor these conditions and will attempt to adjust our business processes, as appropriate, to mitigate these risks to our business.

Our liquidity and capital resources as of September 30, 2016 and December 31, 2015 were as follows (in millions):

|  | September 30,<br>2016 | December 31,<br>2015 | Change   |
|--|-----------------------|----------------------|----------|
| Cash and cash equivalents              | \$ 707.3              | \$ 397.0             | \$ 310.3 |
| Short-term investments                 | 327.5                 | 195.6                | 131.9    |
| Long-term investments                  | 363.0                 | 425.7                | (62.7 )  |
| Cash, cash equivalents and investments | \$ 1,397.8            | \$ 1,018.3           | \$ 379.5 |
| Current assets                         | \$ 1,666.6            | \$ 1,089.6           | \$ 577.0 |
| Current liabilities                    | 377.3                 | 445.5                | (68.2 )  |
| Working capital                        | \$ 1,289.3            | \$ 644.1             | \$ 645.2 |
| Convertible debt                       | \$ 675.6              | \$ 662.3             | \$ 13.3  |

Our cash flows for the nine months ended September 30, 2016 and 2015 are summarized as follows (in millions):

|  | 2016      | 2015      | Change     |
|--|-----------|-----------|------------|
| Cash and cash equivalents at the beginning of the period | \$397.0   | \$875.5   | \$(478.5 ) |
| Net cash used in operating activities                    | (228.2 )  | (153.7 )  | (74.5 )    |
| Net cash used in investing activities                    | (174.4 )  | (1,259.1) | 1,084.7    |
| Net cash provided by financing activities                | 707.8     | 916.2     | (208.4 )   |
| Foreign exchange impact                                  | 5.1       | (2.6 )    | 7.7        |
| Cash and cash equivalents at the end of the period       | \$707.3   | \$376.3   | \$331.0    |
| Short-term and long-term investments                     | 690.5     | 756.4     | (65.9 )    |
| Cash, cash equivalents and investments                   | \$1,397.8 | \$1,132.7 | \$265.1    |

#### Working Capital

Working capital increased by \$645.2 million, from \$644.1 million at December 31, 2015 to \$1,289.3 million at September 30, 2016. The increase in working capital was attributed to the following (in millions):

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|   |           |
|---|-----------|
| Working capital at December 31, 2015                        | \$644.1   |
| Increased cash, cash equivalents and short-term investments | 442.2     |
| Increased accounts receivable, net                          | 50.9      |
| Increased inventory   | 75.7      |
| Decreased current liabilities                               | 68.1      |
| Increased other current assets                              | 8.3       |
| Working capital at September 30, 2016                       | \$1,289.3 |

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The increase in cash, cash equivalents and short-term investments was primarily attributed to \$712.9 million in net proceeds from the August 2016 public offering of our common stock, offset in part by \$228.2 million of cash used to fund operating activities, net cash invested in property, plant, and equipment of \$96.8 million, and \$8.4 million used to hold a purchase of land in escrow. The increase in accounts receivable was attributed to increased revenues and the timing of cash receipts from customers. The increase in inventory was primarily attributed to the manufacture of inventories for all commercial products and pre-launch inventory related to Brineura. The decrease in current liabilities was primarily due to a \$86.4 million decrease in accounts payable and accrued expenses related to payments of R&D expenses and income taxes, offset by the \$22.5 million of convertible notes due in April 2017 changing from noncurrent to current liabilities and a \$4.2 million decrease in the fair value of short-term contingent consideration payable.

Our product sales to government-owned or government-funded customers in certain countries, including Italy, Spain, Portugal, Greece and Russia, are subject to payment terms that are imposed by government authorities. Because these customers are government-owned or government-funded, we may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings, or default in these countries, may decrease the likelihood that we will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to our operating results. Historically we have not experienced a significant level of uncollected receivables and have received continued payments from our more aged accounts. We believe that the allowances for doubtful accounts for these countries are adequate based on our analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. As of September 30, 2016, approximately 12% of our outstanding accounts receivable relate to such countries. See Note 16 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, 2016 was \$228.2 million, compared to cash used in operating activities of \$153.7 million for the nine months ended September 30, 2015. Cash used in operating activities primarily consisted of net loss of \$551.5 million, adjusted for non-cash items such as \$599.1 million asset impairment charge, \$97.2 million for stock-based compensation expenses, \$76.8 million for depreciation and amortization expense, \$22.3 million of non-cash interest expense, offset by \$206.7 million for deferred income taxes benefit and \$57.0 million related to the decrease in the fair value of contingent acquisition consideration payable. Changes in operating assets and liabilities resulted in a net cash outflow of \$200.7 million that consisted primarily of increased cash outflow for R&D expenses and increased inventory spending on the pre-launch Brineura inventory as well as for all commercial products to meet anticipated future sales demand.

#### Cash Used in Investing Activities

Net cash used in investing activities during the nine months ended September 30, 2016 was \$174.4 million, compared to net cash used in investing activities during the nine months ended September 30, 2015 of \$1,259.1 million. The decrease in net cash used in investing activities for the nine months ended September 30, 2016 compared to the nine months ended September 30, 2015 primarily consisted of a \$536.9 million decrease in cash used to acquire a business, a \$513.5 million decrease in net purchases of available-for-sale securities, and a \$27.0 million decrease of capital purchases. We expect to continue to make significant capital investments in our manufacturing facilities 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, 2016 was \$707.8 million, compared to net cash provided by financing activities of \$916.2 million for the nine months ended September 30, 2015. The decrease in net cash provided by financing activities for the nine months ended September 30, 2016 was primarily attributable to a lower amount of net proceeds from public offerings of common stock in 2016 as compared to 2015 of \$175.3 million, a \$33.3 million increase in taxes paid related to net share settlement of employee equity awards, and by a \$2.0 million decrease in proceeds from employee stock option exercises and ESPP purchases.

## Other Information

Our \$772.5 million (undiscounted) of total convertible debt as of September 30, 2016, 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 (of which \$22.5 million, undiscounted, remained outstanding as of September 30, 2016) 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. See Note 11 to our accompanying Condensed Consolidated Financial Statements for additional discussion.

On August 12, 2016, we sold 7.5 million shares of our common stock at a price of \$96.00 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds of approximately \$712.9 million from this public offering after accounting for the underwriting discount and offering costs.

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

On January 15, 2015, we closed the initial offering period related to our offer to purchase all of the ordinary shares of Prosensa (Prosensa Shares), a public limited liability company organized under the laws of the Netherlands, purchasing 93.4% of the Prosensa Shares and immediately launched a subsequent offering period that expired on January 29, 2015. As of the expiration of the subsequent offering period, we paid \$620.7 million for approximately 35 million Prosensa Shares, representing 96.8% of all the outstanding Prosensa Shares. Additionally, we paid approximately \$38.6 million for the options that vested pursuant to the definitive purchase agreement. On February 12, 2015, we completed an asset transfer and we paid \$20.8 million to the remaining Prosensa shareholders. Effective February 12, 2015, Prosensa has been dissolved and was liquidated in January 2016 under Dutch law.

## 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 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 product candidates may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.





Our investment in our product development programs and continued development of our existing commercial products has a major impact on our operating performance. Our R&D expenses of our major development programs from inception to September 30, 2016 were as follows (in millions):

|                                     | Since Program<br>Inception |
|-------------------------------------|----------------------------|
| Vimizim                             | \$ 422.8                   |
| Pegvaliase                          | 371.2                      |
| Brineura                            | 163.4                      |
| Vosoritide                          | 154.3                      |
| Kyndrisa <sup>(1)</sup>             | 125.7                      |
| Reveglucosidase alfa <sup>(1)</sup> | 244.7                      |
| BMN 270                             | 101.1                      |
| NAGLU                               | 87.8                       |
| Other approved products             | 469.8                      |
| Other and non-allocated             | Not meaningful             |

(1) Programs for which internal development has been terminated.

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 our manufacturing capacity; preclinical studies and clinical trials for our other product candidates; potential licenses and other acquisitions of complementary technologies, products and companies; and general corporate purposes.

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

- product sales and profitability of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- manufacturing, supply or distribution of our product candidates and commercial products;
- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;
- results relating to our lawsuits against Par to protect our patents relating to Kuvan and generic competition to Kuvan relating to our settlement with DRL;
- government regulatory action affecting our product candidates or our competitors' drug products in both the U.S. and non-U.S. countries;
- developments or disputes concerning patent or proprietary rights;
- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;
- economic conditions in the U.S. or abroad;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results, including due to timing of large order for our products, in particular in Latin America, where governments place large periodic orders for Vimizim and Naglazyme;
- changes in company assessments or financial estimates by securities analysts;
- acquisitions of products, businesses, or other assets; and
- sales of our shares of stock by us, our significant shareholders, or members of our management or Board of Directors.

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.

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### Contractual and Commercial Obligations

We have contractual and commercial obligations under our debt, operating leases and other obligations related to R&D activities, purchase commitments, licenses and sales royalties with annual minimums. We are also subject to contingent payments related to certain development and regulatory activities and commercial sales and licensing milestones totaling approximately \$754.4 million as of September 30, 2016, which are due upon achievement of certain development and commercial milestones, and if they occur before certain dates in the future. Of this amount, \$207.6 million (USD equivalent of 185 million Euros translated at 1.12 USD per Euro) relates to the Merck PKU Business acquisition and \$58.2 million relates to programs that are no longer being developed. See Note 18 to our accompanying Condensed Consolidated Financial Statements for additional discussion.

There have been no material changes to the Company's contractual and commercial obligations during the nine months ended September 30, 2016, as compared to the significant accounting policies disclosed in Management's Discussion and Analysis in the Company's Annual Report on Form 10-K for the year ended December 31, 2015.

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

Our market risks during the nine months ended September 30, 2016 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, 2015, which was filed with the SEC on February 29, 2016.

### Item 4. Controls and Procedures

#### (a) Controls and Procedures

An evaluation was carried out, under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act), as of the end of the period covered by this report.

Based on the evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures were effective as of September 30, 2016.

In designing and evaluating our disclosure controls and procedures, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management must apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Accordingly, our disclosure controls and procedures are designed to provide reasonable, not absolute, assurance that the objectives of our disclosure controls system are met.

#### (b) Change in Internal Controls over Financial Reporting

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

## PART II. OTHER INFORMATION

Item 1. Legal Proceedings.  
Paragraph IV Notices

We received a paragraph IV notice letter, dated January 22, 2015, from Par, notifying us that Par had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie, on March 6, 2015, we filed a lawsuit against Par in the United States District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

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We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of our patents listed in the FDA's Orange Book. On February 22, 2016, we filed a lawsuit against Par in the United States District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018. In response, Par alleged, *inter alia*, that the asserted patents are not infringed and/or are invalid.

The two cases against Par have been consolidated in the District of New Jersey. The court held a claim construction hearing on May 5, 2016 but has not yet issued its ruling. Fact discovery closed in September 2016 and trial is scheduled for June 2017.

#### SEC Subpoena

In August 2016, we received a subpoena from the staff of the SEC requesting that we produce documents in connection with a non-public, fact-finding inquiry related to our former drisapersen program. The letter enclosing the subpoena states that the investigation and the subpoena do not mean that the Company or anyone else has broken the law, or that the SEC has a negative opinion of any person, entity or security. We intend to cooperate fully with the SEC in this matter. We are not able to predict whether any proceeding may be instituted in connection with the subpoena, or the outcome of any proceeding that may be instituted.

#### 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, 2015, which was filed with the SEC on February 29, 2016.

#### Risks Related to Our Business

\*If we fail to obtain 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. For example, in January

2016, the FDA issued a complete response letter to our New Drug Application for Kyndrisa, concluding that the standard of substantial evidence of Kyndrisa's effectiveness had not been met, and we subsequently decided to discontinue clinical and regulatory development of Kyndrisa as well as the three other first generation follow-on products, BMN 044, BMN 045 and BMN 053 (other exons). Products distributed abroad are also subject to government regulation by international regulatory authorities, such as the EMA. The approval process in the EU and other countries can also be lengthy and expensive and regulatory approval is also never certain.

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Although the FDA and the EMA have programs to facilitate accelerated approval processes, the timelines agreed under legislative goals or mandated by regulations are subject to the possibility of substantial delays. In addition, the FDA, the EMA and other international regulatory authorities have substantial discretion over the approval process for pharmaceutical products. These regulatory agencies may 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 foreign 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.

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

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. 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.

Any product for which we have obtained regulatory approval, or for which we obtain approval in the future, is subject to, or will be subject to, extensive ongoing regulatory requirements by the FDA, the EMA and other comparable international regulatory authorities, and if we fail to comply with regulatory requirements or if we experience unanticipated problems with our approved products, we may be subject to penalties, we will be unable to generate revenue from the sale of such products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.



Naglazyme, Aldurazyme, Kuvan and Vimizim have received regulatory approval to be commercially marketed and sold in the U.S., the EU and other countries. Firdapse has received regulatory approval to be commercially marketed only in the EU. Any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future, along with the manufacturing processes and practices, post-approval clinical research, product labeling, advertising and promotional activities for such product, are subject to continual requirements of, and review by, the FDA, the EMA and other comparable international regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, current good manufacturing practices (cGMP) requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians, import and export requirements and recordkeeping.

Promotional communications with respect to prescription drugs, including biologics, are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we will not be able to promote any products we develop for indications or uses for which they are not approved. We also are subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMP and other regulations.

In addition, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved products. The FDA, the EMA and other 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. Discovery after approval of previously unknown problems with any such products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in actions such as:

- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
  - restrictions on product manufacturing processes;
- restrictions on the marketing of a product;
- restrictions on product distribution;
  - requirements to conduct post-marketing clinical trials;
- untitled or warning letters or other adverse publicity;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- refusal to permit the import or export of our products;
- product seizure;
- fines, restitution or disgorgement of profits or revenue;
- injunctions; or
- imposition of civil or criminal penalties.

If such regulatory actions are taken, the value of our company and our operating results will be adversely affected. Additionally, if the FDA, the EMA or any other comparable international regulatory agency withdraws its approval of a product, we will be unable to generate revenue from the sale of that product in the relevant jurisdiction, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased. Accordingly, we continue to expend significant time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance, post-marketing studies and quality control.

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

As part of our business strategy, we have developed and may in the future 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. In the EU, orphan drug designation is granted to drugs intended to treat a rare disease or condition, defined as having a prevalence of no more than five in 10,000 people in the EU, which is equivalent to around 250,000 people or fewer. 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 product candidates and even if we obtain orphan drug designation for our future product candidates, due to the uncertainties associated with developing biopharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication, which means that we may not obtain orphan drug exclusivity and could also potentially be blocked from approval of certain product candidates until the competitor product's orphan drug exclusivity period expires. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition and the same drug can be approved for different conditions and potentially used off-label in the orphan indication. Even after an orphan drug is approved and granted orphan drug exclusivity, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer or 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 biosimilars approved through an abbreviated regulatory pathway.

Our Naglazyme, Aldurazyme and Vimizim products are regulated by the FDA as biologics under the Federal Food, Drug, and Cosmetic Act (the FDC Act) and the Public Health Service Act (the PHS Act). Biologics require the submission of a biologics license application (BLA) and approval by the FDA prior to being marketed in the U.S. Historically, a biologic product approved under a BLA was not subject to the generic drug review and approval provisions of the FDC Act. However, the Biologics Price Competition and Innovation Act of 2009 (BPCIA) created a regulatory pathway under the PHS Act for the abbreviated approval of 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 BPCIA establishes a period of 12 years of data exclusivity for reference products, which protects the data in the original BLA by prohibiting sponsors of biosimilars from gaining FDA approval based in part on reference to data in the original BLA. Aldurazyme's data exclusivity under the BPCIA expired in 2015, Naglazyme's data exclusivity under the BPCIA expires in 2017, and Vimizim's data exclusivity under the BPCIA expires in 2026. Our products approved under BLAs, as well as products in development that may be approved under BLAs in the future, could be reference products for biosimilar marketing applications.

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

As part of the drug development process we must conduct, at our own expense, preclinical studies in the laboratory, including studies in animals, 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, new drugs for diseases or conditions that affect larger patient populations, are less severe, or are treatable by alternative strategies must be validated through additional preclinical and clinical trials and/or clinical trials with higher enrollments. With respect to our early stage drug candidates, we may need to perform multiple

preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays to our development timeline. Furthermore, even if we obtain favorable results in preclinical studies, the results in humans may be significantly different. After we have conducted preclinical studies, we must demonstrate that our drug products are safe and efficacious for use in the targeted human patients in order to receive regulatory approval for commercial sale. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, and favorable data from interim analyses do not ensure the final results of a trial will be favorable. Product candidates may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials, or despite having favorable data in connection with an interim analysis. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials.

Specific factors that could cause delay or termination of our clinical trials or stop us from filing for regulatory approval of our product candidates include the following:

- slow or insufficient patient enrollment;

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- slow recruitment of, and completion of necessary institutional approvals at, clinical sites;
- budgetary constraints or prohibitively high clinical trial costs;
- longer treatment time required to demonstrate efficacy;
- lack of sufficient supplies of the product candidate;
- adverse medical events or side effects in treated patients, including immune reactions;
- lack of effectiveness of the product candidate being tested;
- regulatory requests for additional clinical trials or pre-clinical studies;
- deviations in standards for Good Clinical Practice (GCP); and
- disputes with or disruptions in our relationships with clinical trial partners, including CROs, clinical laboratories, clinical sites, and principal investigators.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services reportable to the FDA or other regulatory authority. If the FDA or other regulatory authority concludes that a financial relationship between us and a principal investigator has created a conflict of interest, the FDA or other regulatory authority may question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized.

\*Our BMN 270 program is based on a gene therapy approach, which, as a novel technology, presents additional treatment, regulatory, manufacturing, and commercial risks in relation to our other, more traditional drug development programs.

In relation to our other drug products, gene therapy products, such as our product candidate BMN 270, present unique treatment risks. The goal of gene therapy is to be able to correct an inborn genetic defect through one-time administration of therapeutic genetic material containing non-defective gene copies. The gene copies are designed to reside permanently in a patient, allowing the patient to produce an essential protein or ribonucleic acid (RNA) molecule that a healthy person would normally produce. There is a risk, however, that the new gene copies will produce too much or too little of the desired protein or RNA. There is also a risk that production of the desired protein or RNA will increase or decrease over time. Because the treatment is irreversible, there may be challenges in managing side effects, particularly those caused by overproduction. Adverse effects would not be able to be reversed or relieved by stopping dosing, and we may have to develop additional clinical safety procedures. Furthermore, because the new gene copies are designed to reside permanently in a patient, there is a risk that they will disrupt other normal biological molecules and processes, including other healthy genes, and we may not learn the nature and magnitude of these side effects until long after clinical trials have been completed.

We may experience development problems related to our gene therapy program that cause significant delays or unanticipated costs, or that cannot be solved. Given the few precedents of approved gene therapy products, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidate in any jurisdiction. Regulatory requirements governing gene and cell therapy products have evolved and may continue to change in the future. Regulatory review agencies and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our treatment candidate or lead to significant post-approval studies, limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring BMN 270 to market could have a negative effect on our business and financial condition. Even if we do obtain regulatory approval, ethical, social and legal concerns about gene therapy arising in the future could result in additional regulations restricting or prohibiting sale of our product.

Even if we obtain regulatory approval for BMN 270, we may experience delays, and increased costs, in developing a sustainable, reproducible and large-scale manufacturing process. Gene therapy products are novel, complex and difficult to manufacture, and have, only in limited cases, been manufactured at scales sufficient for pivotal trials and

commercialization. Few pharmaceutical contract manufacturers specialize in gene therapy products and those that do are still developing appropriate processes and facilities for large-scale production. Whether we produce BMN 270 at a contract manufacturer or develop our own gene therapy manufacturing capability, we will likely face technical and scientific challenges, considerable capital costs, and potential difficulty in recruiting and hiring experienced, qualified personnel. As a result, we could experience manufacturing delays that prevent us from completing our clinical studies or commercializing BMN 270 in a timely, or on a profitable, basis, if at all.

Due to the relative novelty of gene therapy and the potential to provide therapeutic treatment with a one-time administration, we also face uncertainty with respect to the pricing, coverage and reimbursement of BMN 270, if approved. In order to recover our research and development costs and commercialize a one-time treatment, such as BMN 270, on a profitable basis, we expect the cost of a single administration of BMN 270 to be substantial. Therefore, we expect that coverage and reimbursement by governments and other third-party payors will be essential for the vast majority of patients to be able to afford BMN 270. Accordingly, sales of BMN 270, if approved, will depend substantially, both domestically and internationally, on the extent to which its cost will be paid by third-party payors. Even if coverage is provided, the reimbursement amounts approved by third-party payors may not be high enough to allow us to realize a sufficient return on our investment.

We also face uncertainty as to whether gene therapy will gain the acceptance of the public or the medical community. Even if we obtain regulatory approval for BMN 270, the commercial success of BMN 270 will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidate in particular, as medically necessary, cost-effective and safe. In particular, our success will depend upon physicians prescribing our product candidate in lieu of existing treatments they are already familiar with and for which greater clinical data may be available. Even if BMN 270 displays a favorable efficacy and safety profile in clinical trials and is ultimately approved, market acceptance of BMN 270 will not be fully known until after it is launched. Negative public opinion or more restrictive government regulations or could have a negative effect on our business and financial condition and may delay or impair the development and commercialization of, and demand for, BMN 270.

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

Since we began operations in March 1997, we have been engaged in substantial research and development and capital investments, and we have operated at a net loss for each year since our inception, with the exception of 2008 and 2010. Based upon our current plan for investments in research and development for existing and new programs, as well as capital investments in our facilities and working capital such as inventory, we expect to operate at a net loss and experience net cash outflows for at least the next 12 months. Our future profitability and cash flows depend 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, the impact of any possible future business development transactions and other risks set forth in this Risk Factors section. The extent of our future losses and the timing of profitability and positive cash flows are highly uncertain. If we fail to become profitable and cash flow positive or are unable to sustain profitability and positive cash flows on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

\*If we fail to 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, 2016, we had cash, cash equivalents and short and long-term investments totaling \$1.4 billion and long-term debt obligations of \$772.5 million (undiscounted). In January 2016 we terminated our License and Commercialization Agreement with Ares Trading, S.A. (Merck Serono). Pursuant to the Termination and Transition Agreement related to Kuvan and the Termination Agreement related to pegvaliase, we made cash payments on this transaction totaling \$374.5 million in the nine months ended September 30, 2016, and may pay Merck Serono up to a maximum of €60 million, in cash, if future sales milestones are met with respect to Kuvan and up to a maximum of €125 million, in cash, if future development milestones are met with respect to pegvaliase. In October 2013, we completed an offering of senior subordinated convertible notes and received net proceeds of approximately \$696.4 million, after

deducting commissions, estimated offering expenses payable by us and the purchase of the related capped calls. We will need cash to not only repay the principal amount of our 1.875% senior subordinated convertible notes due 2017 (the 2017 Notes), 0.75% senior subordinated convertible notes due 2018 (the 2018 Notes) and 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes and, together with the 2017 Notes and 2018 Notes, the Notes) but also the ongoing interest due on the Notes during their term.

We will require additional financing to fund the repayment of our Notes, future milestone payments and 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 due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise any necessary additional financing we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

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

- our ability to successfully market and sell Vimizim, Naglazyme, Kuvan and Firdapse;
- Genzyme's ability to continue to successfully commercialize Aldurazyme;



- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the progress of research programs carried out by us;
- our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD Therapeutics, Inc., ZyStor Therapeutics, Inc., Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc., and under the termination agreements with Merck Serono related to Kuvan and pegvaliase milestones;
- 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 or arrangements.

We have in the past raised capital through the public offering of our common stock, and in August 2016 we completed a public offering of 7,500,000 shares of our common stock at a price of \$96.00 per share, resulting in net proceeds of approximately \$712.9 million. We will 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 will 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.

\*We have incurred substantial indebtedness that may decrease our business flexibility, access to capital, and/or increase our borrowing costs, which may adversely affect our operations and financial results.

As of September 30, 2016, we had \$772.5 million (undiscounted) principal amount of indebtedness, including \$375.0 million (undiscounted) of indebtedness under the 2018 Notes and \$375.0 million (undiscounted) principal amount of indebtedness under the 2020 Notes. Our indebtedness may:

- limit our ability to borrow additional funds for working capital, capital expenditures, acquisitions or other general business purposes;
- limit our ability to use our cash flow or obtain additional financing for future working capital, capital expenditures, acquisitions or other general business purposes;
- require us to use a substantial portion of our cash flow from operations to make debt service payments;
- limit our flexibility to plan for, or react to, changes in our business and industry;
- place us at a competitive disadvantage compared to our less leveraged competitors; and
- increase our vulnerability to the impact of adverse economic and industry conditions.

In addition, our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time.

Our indebtedness consists primarily of the 2018 and 2020 Notes, which, if not converted, will be required to be repaid in cash at maturity in 2018 and 2020. In addition, in the event the conditional conversion feature of the Notes is triggered, holders of Notes will be entitled to convert the Notes at any time during specified periods at their option.

We intend to settle the principal amount of our conversion obligation in cash, which could adversely affect our liquidity. Even if holders do not elect to convert their Notes, we could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of the Notes as a current rather than long-term liability, which would result in a material reduction of our net working capital. Moreover, if we are unable to refinance

the Notes, we must repay the Notes. While we could seek to obtain third-party financing to pay for any amounts due in cash upon such events, we cannot be sure that such third-party financing will be available on commercially reasonable terms, if at all. Furthermore, if we are required to share settle any conversions of Notes, due to lack of requisite liquidity or otherwise, we may cease to be eligible to account for the Notes using the treasury stock method, which may adversely impact our diluted earnings per share.

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

Before we can begin commercial manufacture of our products, regulatory authorities must approve marketing applications that identify manufacturing facilities operated by us or our contract manufacturers that have passed regulatory inspection and manufacturing processes that are acceptable to the regulatory authorities. In addition, our pharmaceutical manufacturing facilities are continuously subject to inspection by the FDA and international regulatory authorities, before and after product approval. Our manufacturing facility in the U.S. has been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Aldurazyme, Naglazyme and Vimizim. Our manufacturing facility in Shanbally, Cork, Ireland has been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Vimizim. In addition, our third-party manufacturers' facilities involved with the manufacture of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse have also been inspected and approved by various regulatory authorities. Although we are not involved in the day-to-day operations of our contract manufacturers, we are ultimately responsible for ensuring that our products are manufactured in accordance with cGMP regulations.

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

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

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

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

The development of commercially viable manufacturing processes typically is very difficult to achieve and is often very expensive and may require extended periods of time. Changes in manufacturing processes (including manufacturing cell lines), equipment or facilities may require us to complete clinical trials to receive regulatory approval of any manufacturing improvements.

Also, we may be required to demonstrate product comparability between a biological product made after a manufacturing change and the product made before implementation of the change through additional types of

analytical and functional testing or may have to complete additional clinical studies. If we contract for manufacturing services with an unproven process, our contractor is subject to the same uncertainties, high standards and regulatory controls, and may therefore experience difficulty if further process development is necessary.

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

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

Although we have entered into contractual relationships with third-party manufacturers to produce the active ingredient in Kuvan and Firdapse, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for Kuvan and Firdapse or sell these products at all, we may lose potential revenue, and we may be forced to terminate a program. 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, Aldurazyme and Vimizim. If those manufacturers are unwilling or unable to fulfill their contractual obligations or satisfy demand outside of or in excess of the contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. Further, the availability of suitable contract manufacturing capacity at scheduled or optimum times is not certain.

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

We manufacture Naglazyme, Aldurazyme and a portion of Vimizim in a manufacturing facility 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 manufacturers' ability to manufacture Kuvan or Firdapse.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Naglazyme and Aldurazyme and is one of two manufacturing facilities for 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.

We depend on single-source suppliers for critical raw materials and a limited number of manufacturing facilities to manufacture our finished products and product candidates. Numerous factors could cause interruptions in the supply or manufacture of our products and product candidates, including:

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

•conditions affecting the cost and availability of raw materials.

If one of our suppliers or manufacturers fails or refuses to supply us with necessary raw materials or finished products or product candidates on a timely basis or at all, it would take a significant amount of time and expense to qualify a new supplier or manufacturer. We may not be able to obtain active ingredients or finished products from new suppliers or manufacturers on acceptable terms and at reasonable prices, or at all.

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 in particular we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in each disease population is 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 payors, 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 payors. 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 payors, 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 payor, 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.

Government authorities and other third-party payors are developing increasingly sophisticated methods of controlling healthcare costs, such as by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices as a condition of coverage, are using restrictive formularies and preferred drug lists to leverage greater discounts in competitive classes, and are challenging the prices charged for medical products. Further, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payors in the U.S. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

We cannot be sure that coverage and reimbursement will be available for any product that we commercialize or will continue to be available for any product that we have commercialized and, if reimbursement is available, what the level of reimbursement will be. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may not successfully commercialize any product candidate for which we obtain marketing approval or continue to market any product that has already been commercialized.

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

concessions over time.

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

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

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

These programs are not well defined in some countries and are subject to changes in requirements and funding levels. Any change to these programs could adversely affect our ability to sell our products in those countries and delay sales. If the programs are not funded by the respective government, there could be insufficient funds to pay for all patients. Further, governments have in the past undertaken and may in the future undertake unofficial measures to limit purchases of our products, including initially denying



coverage for purchasers, delaying orders and denying or taking excessively long to approve customs clearance. Any such actions could materially delay or reduce our revenues from such countries.

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

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

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

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

We expect that coverage and reimbursement may be increasingly restricted both in the U.S. and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. In particular, drug pricing by pharmaceutical companies has recently come under increased scrutiny and continues to be subject to intense political and public debate in the U.S. and abroad. Governmental and private third-party payors 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. Specifically, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. 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 payors, 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 our products. 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 or negative publicity related to our drug pricing or the pricing of pharmaceutical drugs generally could restrict the amount that we are able to charge for our current and future products or our sales volume, which would adversely affect our revenue and results of operations.

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

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

Several provisions of the law have affected us and increased 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, and created a new Patient-Centered Outcomes Research Institute to oversee clinical effectiveness research.

In addition, other legislative changes have been adopted since the PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, following passage of the Bipartisan Budget Act of 2015, will remain in effect through 2025 unless additional Congressional action is taken. In January

2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

We anticipate that the PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and an additional downward pressure on the reimbursement our customers may receive for our products. Further, there have been judicial and Congressional challenges to certain aspects of the PPACA, and we expect there will be additional challenges and amendments to the PPACA in the future. 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 health care 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 laws and regulations, including anti-kickback laws, false claims laws, data privacy and security laws, and laws related to ensuring compliance. The federal Anti-Kickback Statute makes it illegal for any person or entity, including a pharmaceutical company, to knowingly and willfully offer, solicit, pay or receive any remuneration, directly or indirectly, in exchange for or to induce the referral of business, including the purchase, order or prescription of a particular drug, for which payment may be made under federal health care programs, such as Medicare and Medicaid. Under federal government regulations, certain arrangements, or safe harbors, are deemed not to violate the federal Anti-Kickback Statute. However, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration not intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from Anti-Kickback liability, although we seek to comply with these safe harbors. 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 payors.

Federal and state false claims laws, including the civil False Claims Act, 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, or knowingly making, using, or causing to be made or used, a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government. 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 (HIPAA), we also are prohibited from knowingly and willfully executing a scheme to defraud any health care benefit program, including private payors, 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.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of individuals and entities, with respect to safeguarding the privacy, security and transmission of individually identifiable health information. Many state and foreign laws also govern the privacy and security of health information. They often differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Substantial new provisions affecting compliance have also been adopted, which may require us to modify our business practices with health care practitioners. The PPACA, through the Physician Payments Sunshine Act, requires drug manufacturers to collect and report to CMS information on payments or transfers of value to physicians and teaching hospitals, as well as investment and ownership interests held by physicians and their immediate family members during the preceding calendar year. Failure to submit required information may result in civil monetary penalties.

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 Pharmaceutical Research and Manufacturers of America (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.

Due to 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, our business activities could be subject to challenge under one or more of such laws. For example, in August 2016, we received a subpoena from the staff of the SEC requesting that we produce documents in connection with a non-public, fact-finding inquiry related to our former drisapersen program. The letter enclosing the subpoena states that the investigation and the subpoena do not mean that the Company or anyone else has broken the law, or that the SEC has a negative opinion of any person, entity or security. We intend to cooperate fully with the SEC in this matter. We are not able to predict whether any proceeding may be instituted in connection with the subpoena, or the outcome of any proceeding that may be instituted.

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 Statute and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them in order to commit a violation. 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 damages, fines, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, curtailment of our operations, 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, Kuvan, Naglazyme and Vimizim, and all of the sales of Firdapse are generated from countries other than the U.S. We have operations in Canada and in several European, Middle Eastern, Asian, and Latin American 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:

- the increased complexity and costs inherent in managing international operations;
- diverse regulatory and compliance requirements, and changes in those 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;
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors' and service providers' activities that may fall within the purview of the Foreign Corrupt Practices Act (the FCPA); and
- regulations relating to data security and the unauthorized use of, or access to, commercial and personal information.

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

As we continue to expand our existing international operations, we may encounter new risks. For example, as we focus on building our international sales and distribution networks in new geographic regions, we must continue to

develop relationships with qualified local distributors and trading companies. If we are not successful in developing and maintaining these relationships, we may not be able to grow sales in these geographic regions. These or other similar risks could adversely affect our revenue and profitability.

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

A significant and growing portion of our revenues and earnings, as well as our substantial international net assets, are exposed to changes in foreign exchange rates. As we operate in multiple foreign currencies, including the euro, the Brazilian real, the U.K. pound, the Canadian dollar, the Swiss franc, the Japanese yen and several other currencies, changes in those currencies relative to the U.S. dollar will impact our revenues and expenses. If the U.S. dollar were to weaken against another currency, assuming all other variables

remained constant, our revenues would increase, having a positive impact on earnings, and our overall expenses would increase, having a negative impact on earnings. Conversely, if the U.S. dollar were to strengthen against another currency, assuming all other variables remained constant, our revenues would decrease, having a negative impact on earnings, and our overall expenses would decrease, having a positive impact on earnings. In addition, because our financial statements are reported in U.S. dollars, changes in currency exchange rates between the U.S. dollar and other currencies have had, and will continue to have, an impact on our results of operations. Therefore, significant changes in foreign exchange rates can impact our results and our financial guidance.

We implement currency hedges intended to reduce our exposure to changes in foreign currency exchange rates. However, our hedging strategies may not be successful, and any of our unhedged foreign exchange exposures will continue to be subject to market fluctuations. These risks could cause a material adverse effect on our business, financial position and results of operations and could cause the market value of our common stock to decline.

If we are unable to protect our intellectual property, we may not be able to compete effectively.

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

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

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

- With respect to pending patent applications, unless and until actually issued, the protective value of these applications is impossible to determine. We do not know whether our patent applications will result in issued patents.
- Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us or that they filed their application for a patent on a claimed invention before we did. Competitors may also claim that we are infringing on their patents and therefore we cannot practice our technology. Competitors may also contest our patents by showing the patent examiner or a court that the invention was not original, was not novel or was obvious, for example. In litigation, a competitor could claim that our issued patents are not valid or are unenforceable for a number of reasons. If a court agrees, we would not be able to enforce that patent. We have no meaningful experience with competitors interfering with or challenging the validity or enforceability of our patents or patent applications.
- Generic manufacturers may use litigation and regulatory means to obtain approval for generic versions of our products.
- Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing products, which could increase our operating expenses and delay product programs. We may not have the financial ability to sustain a patent infringement action, or it may not be financially reasonable to do so.

Receipt of a patent may not provide much, if any, practical protection. For example, if we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent.

The Leahy-Smith America Invents Act of 2011, which reformed certain patent laws in the U.S., may create additional uncertainty. Among the significant changes are switching from a “first-to-invent” system to a “first-to-file” system, and the implementation of new procedures that permit competitors to challenge our patents in the U.S. Patent and Trademark Office after grant.

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



Moreover, there is an increasing trend in the EU requiring public disclosure of development data, in particular clinical trial data. These data were traditionally regarded as confidential commercial information; however, under policies recently adopted in the EU, data submitted to the EMA in MAAs may be subject to public disclosure. Exactly how the new disclosure policy will be implemented is unclear; however, it could result in the EMA's public disclosure of certain of our clinical study reports, including pre-clinical data, and patient level data. The move toward public disclosure of development data could adversely affect our business in many ways, including, for example, resulting in the disclosure of our confidential methodologies for pre-clinical and clinical development of our products, preventing us from obtaining intellectual property right protection for innovations, requiring us to allocate significant resources to prevent other companies from violating our intellectual property rights, adding even more complexity to processing health data from clinical trials consistent with applicable data privacy regulations, and enabling competitors to use our data to gain approvals for their own products.

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

- Defending a lawsuit takes significant executive resources and can be very expensive.

- If a court decides that our product infringes a competitor's intellectual property, we may have to pay substantial damages.

- With respect to patents, in addition to requiring us to pay substantial damages, a court may prohibit us from making, selling, offering to sell, importing or using our product unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, it may not be available on commercially reasonable terms. For example, we may have to pay substantial royalties or grant cross licenses to our patents and patent applications.

- We may need to redesign our product so it does not infringe the intellectual property rights of others.

- Redesigning our product so it does not infringe the intellectual property rights of competitors may not be possible or could require substantial funds and time.

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

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

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

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

If the MMS Agreement is terminated for breach, the breaching party will transfer its interest in the BioMarin/Genzyme 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 BioMarin/Genzyme 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 BioMarin/Genzyme LLC at a specified buyout amount. If such option is not exercised, all rights to Aldurazyme will be sold and the

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

If we fail to develop new products and product candidates or compete successfully with respect to acquisitions, joint ventures, licenses or other collaboration opportunities, our ability to continue to expand our product pipeline and our growth and development would be impaired.

Our future growth and development depends in part on our ability to successfully develop new products from our research and development activities. The development of biopharmaceutical products is very expensive and time intensive and involves a great degree of risk. The outcomes of research and development programs, especially for innovative biopharmaceuticals, are inherently uncertain and may not result in the commercialization of any products.

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

Universities and public and private research institutions also compete with us. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our product candidates. We will attempt to license this proprietary technology, if

available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

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

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

We own several patents that cover Kuvan, and we have listed those patents in conjunction with that product in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Hatch-Waxman Act requires an ANDA

applicant seeking FDA approval of its proposed generic product prior to the expiration of our Orange Book-listed patents to certify that the applicant believes that our patents are invalid or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify us of such certification (a paragraph IV notice). Upon receipt of a paragraph IV notice, the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the generic's application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA may approve the ANDA if it is otherwise ready for approval. 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. In addition to our patent protection, we have received three-year Hatch-Waxman exclusivity for a New Patient Population for Kuvan that expires in October 2017, including pediatric exclusivity. Thus, depending on the proposed labeling of a generic product, generic versions of Kuvan may be prohibited until October 2017, though it is possible that an ANDA applicant could propose to carve out information in the Kuvan labeling protected by the New Patient Population exclusivity and obtain approval earlier.

We received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par had filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie, on March 6, 2015, we filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of our patents listed in the FDA's Orange Book. On February 22, 2016, we filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

The two cases against Par have been consolidated in the District of New Jersey. The court held a claim construction hearing on May 5, 2016 but has not yet issued its ruling. Fact discovery closed in September 2016 and trial is scheduled for June 2017.

In September 2015, we entered into a settlement agreement with DRL that resolved patent litigation with DRL in the U.S. related to DRL's ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances.

The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending.

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

We also face potential generic competition for Kuvan in certain foreign countries, including, without limitation, South Korea, Taiwan, and Turkey. Our ability to successfully market and sell Kuvan in many countries in which we operate is based upon patent rights or certain regulatory forms of exclusivity, or both. The scope of our patent rights and regulatory exclusivity for Kuvan vary from country to country and are dependent on the availability of meaningful legal remedies in each country. If our patent rights and regulatory exclusivity for Kuvan are successfully challenged, expire, or otherwise terminate in a particular country, the resulting generic competition could have a material adverse effect on our revenue and results of operations.

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

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. From time to time, we publicly announce the expected

timing of some of these milestones. All of these milestones are based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. If we do not meet these milestones as publicly announced, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

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

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

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

Our success depends on our ability to manage our growth.

Product candidates that we are currently developing or may acquire in the future may be intended for patient populations that are significantly larger than any of 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, Vimizim, and Aldurazyme in MPS diseases, could be greatly reduced. Moreover, if we obtain regulatory approval for BMN 270, the commercial success of BMN 270 will still depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidate in particular, as medically necessary, cost-effective and safe. Changes in treatment method can be caused by the introduction of other companies' products or the development of new technologies or surgical procedures which may not directly compete with ours, but which have the effect of changing how doctors decide to treat a disease.

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

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

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

We rely significantly on our information technology and manufacturing infrastructure to effectively manage and maintain our inventory and internal reports, to manufacture and ship products to customers and to timely invoice them. Any failure, inadequacy or interruption of that infrastructure or security lapse of that technology, including cybersecurity incidents, could harm our ability to operate our business effectively. Our ability to manage and maintain our inventory and internal reports, to manufacture and ship our



products to customers and timely invoice them depends significantly on our enterprise resource planning, production management and other information systems. Cybersecurity attacks in particular are evolving and include, but are not limited to, malicious software, attempts to gain unauthorized access to data and other electronic security breaches that could lead to disruptions in systems, misappropriation of our confidential or otherwise protected information and corruption of data. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access of these systems, may affect our ability to manage and maintain our inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary and confidential information, including research or clinical data, could require significant capital investments to remediate and could adversely affect our business, financial condition and results of operations.

\*Our business is affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates and foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to pass 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 the nine months ended September 30, 2016, 7% of our net product revenues were from Italy, Spain, Portugal, Greece and Russia. Approximately 12% of our total accounts receivable as of September 30, 2016, are related to these countries. If the financial conditions of these countries continues to decline, a substantial portion of the receivables may be uncollectable, which would mean we would have to provide for additional allowances for doubtful accounts or cease selling products in these countries, either of which could adversely affect our results of operations. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

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

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

We expect that many investors in, and potential purchasers of, the Notes will employ, or seek to employ, a convertible arbitrage strategy with respect to the Notes. Investors would typically implement such a strategy by selling short the common stock underlying the Notes and dynamically adjusting their short position while continuing to hold the Notes.

Investors may also implement this type of strategy by entering into swaps on our common stock in lieu of or in addition to short selling the common stock.

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

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

#### 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 have no meaningful relationship to current or historical earnings, asset values, book value or many other criteria based on conventional measures of stock value. The market price of our common stock will fluctuate due to factors including:

- product sales and profitability of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- manufacturing, supply or distribution of our product candidates and commercial products;
- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;
- results relating to our lawsuits against Par to protect our patents relating to Kuvan and generic competition to Kuvan relating to our settlement with DRL;
- government regulatory action affecting our product candidates or our competitors' drug products in both the U.S. and non-U.S. countries;
- developments or disputes concerning patent or proprietary rights;
- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;
- economic conditions in the U.S. or abroad;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results, including due to timing of large order for our products, in particular in Latin America, where governments place large periodic orders for Vimizim and Naglazyme;
- changes in company assessments or financial estimates by securities analysts;
- acquisitions of products, businesses, or other assets; and
- sales of our shares of stock by us, our significant shareholders, or members of our management or Board of Directors.

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.

Conversion of the Notes will dilute the ownership interest of existing stockholders, including holders who had previously converted their Notes, or may otherwise depress the price of our common stock.

The conversion of some or all of the Notes will dilute the ownership interests of existing stockholders to the extent we deliver shares upon conversion of any of the Notes. The Notes may become in the future convertible at the option of their holders prior to their scheduled terms under certain circumstances. Any sales in the public market of the common stock issuable upon such conversion could adversely affect prevailing market prices of our common stock. In addition, the existence of the Notes may encourage short selling by market participants because the conversion of the Notes could be used to satisfy short positions, or anticipated conversion of the Notes into shares of our common stock could depress the price of our common stock.

The capped call transactions may affect the value of the Notes and our common stock.

In connection with the issuance of the 2018 Notes and 2020 Notes, we entered into capped call transactions with respect to 50% of the principal amount of the 2018 Notes and 50% of the principal amount of the 2020 Notes with certain hedge counterparties. The capped call transactions will cover, subject to customary anti-dilution adjustments, the aggregate number of shares of common stock underlying 50% of the principal amount of the relevant Notes and are expected generally to reduce potential dilution to the common stock upon conversion of the relevant Notes in excess of the principal amount of such converted Notes. In connection with establishing their initial hedges of the

capped call transactions, the hedge counterparties (or their affiliates) entered into various derivative transactions with respect to the common stock concurrently with, and/or purchased the common stock shortly after, the pricing of the relevant notes. The hedge counterparties (or their affiliates) are likely to modify their hedge positions by entering into or unwinding various derivative transactions with respect to the common stock and/or by purchasing or selling the common stock or other securities of ours in secondary market transactions prior to the maturity of the relevant Notes (and are likely to do so during the

settlement averaging period under the relevant capped call transactions, which precedes the maturity date of the relevant Notes, and on or around any earlier conversion date related to a conversion of the relevant Notes).

The effect, if any, of any of these transactions and activities on the market price of our common stock or the Notes will depend in part on market conditions and cannot be ascertained at this time, but any of these activities could adversely affect the value of our common stock, which could affect the value of the Notes and the value of our common stock, if any, that Note holders receive upon any conversion of the Notes.

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

The fundamental change repurchase feature of the Notes may delay or prevent an otherwise beneficial attempt to take over our company.

The terms of the Notes require us to repurchase the Notes in the event of a fundamental change. A takeover of our company would trigger options by the respective holders of the applicable Notes to require us to repurchase such Notes. This may have the effect of delaying or preventing a takeover of our company that would otherwise be beneficial to our stockholders or investors in the Notes.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures  
None.

Item 5. Other Information.  
None.

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Item 6. Exhibits.

- 2.1 Purchase Agreement, dated as of November 23, 2014, among BioMarin Falcons B.V., BioMarin Pharmaceutical Inc. and Prosensa Holding N.V., previously filed with the SEC on November 26, 2014 as Exhibit 2.01 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated by reference herein.
- 2.2^ Asset Purchase Agreement between BioMarin Pharmaceutical Inc. and Medivation, Inc., dated August 21, 2015, previously filed with the SEC on October 7, 2015 as Exhibit 2.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 2.3^ Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on February 29, 2016 as Exhibit 2.3 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
- 2.4^ Termination Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on February 29, 2016 as Exhibit 2.4 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
- 2.5^ Termination and Transition Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on February 29, 2016 as Exhibit 2.5 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
- 3.1 Amended and Restated Certificate of Incorporation of BioMarin Pharmaceutical Inc., as amended, previously filed with the SEC on August 8, 2016 as Exhibit 3.1 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.
- 3.2 Amended and Restated Bylaws of BioMarin Pharmaceutical Inc., previously filed with the SEC on June 15, 2015 as Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 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

^ The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.

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, 2016 and December 31, 2015, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three and nine months ended September 30, 2016 and 2015, (iii) Condensed Consolidated Statement of Stockholders' Equity for the nine months ended September 30, 2016, (iv) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2016 and 2015, and (v) Notes to Condensed Consolidated Financial Statements.



SIGNATURE

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

BIOMARIN PHARMACEUTICAL INC.

Dated: November 3, 2016 By /S/ DANIEL SPIEGELMAN  
Daniel Spiegelman,

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

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