

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, 2019
Or

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from _____ to _____ .
Commission File Number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of
incorporation or organization)

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

770 Lindaro Street San Rafael California
(Address of principal executive offices)

94901
(Zip Code)

(415) 506-6700
(Registrant's telephone number including area code)

Securities registered pursuant to Section 12(b) of the Act:

| Title of each class | Trading Symbol(s) | Name of each exchange on which registered |
|---------------------------------|-------------------|---|
| Common Stock, par value \$0.001 | BMRN | The NASDAQ Global Select Market |

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 every Interactive Data File required to be submitted 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 such files). Yes No

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

| | | | |
|-------------------------|-------------------------------------|---------------------------|--------------------------|
| Large Accelerated Filer | <input checked="" type="checkbox"/> | Accelerated Filer | <input type="checkbox"/> |
| Non-accelerated Filer | <input type="checkbox"/> | Smaller Reporting Company | <input type="checkbox"/> |
| Emerging Growth Company | <input type="checkbox"/> | | |

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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: 179,625,303 shares of common stock, par value \$0.001, outstanding as of October 18, 2019.

BIOMARIN PHARMACEUTICAL INC.

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Unless the context suggests otherwise, references in this Quarterly Report on Form 10-Q to “BioMarin,” the “Company,” “we,” “us,” and “our” refer to BioMarin Pharmaceutical Inc. and, where appropriate, its wholly owned subsidiaries.

BioMarin®, Brineura®, Firdapse®, Kuvan®, Naglazyme®, Palynziq® and Vimizim® are our registered trademarks. Aldurazyme® 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.

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,” “could,” “would,” “projects,” “continues,” “estimates,” “potential,” “opportunity” or the negative versions of these terms and other similar expressions. 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, 2018, which was filed with the Securities and Exchange Commission (the SEC) on February 28, 2019. 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 the Company’s management based on information currently available to management and should be considered in connection with any written or oral forward-looking statements that the Company may issue in the future as well as other cautionary statements the Company has made and may make. Except as required by law, the Company does 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 discussion of the Company’s financial condition and results of operations should be read in conjunction with the Company’s Condensed Consolidated Financial Statements and the related Notes thereto included in this Quarterly Report on Form 10-Q.

PART I. FINANCIAL INFORMATION**Item 1. Financial Statements**

BIOMARIN PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
September 30, 2019 and December 31, 2018
(In thousands, except share amounts)

| ASSETS | September 30, 2019 | December 31, 2018 ⁽¹⁾ |
|--|-------------------------------|---|
| | (unaudited) | |
| Current assets: | | |
| Cash and cash equivalents | \$ 423,220 | \$ 493,982 |
| Short-term investments | 297,572 | 590,326 |
| Accounts receivable, net | 402,970 | 342,633 |
| Inventory | 609,049 | 530,871 |
| Other current assets | 126,548 | 98,403 |
| Total current assets | 1,859,359 | 2,056,215 |
| Noncurrent assets: | | |
| Long-term investments | 431,804 | 235,864 |
| Property, plant and equipment, net | 969,300 | 948,682 |
| Intangible assets, net | 462,849 | 491,808 |
| Goodwill | 197,039 | 197,039 |
| Deferred tax assets | 525,131 | 460,952 |
| Other assets | 112,646 | 36,568 |
| Total assets | \$ 4,558,128 | \$ 4,427,128 |
| LIABILITIES AND STOCKHOLDERS' EQUITY | | |
| Current liabilities: | | |
| Accounts payable and accrued liabilities | \$ 483,745 | \$ 437,290 |
| Short-term contingent consideration | 10,000 | 85,951 |
| Total current liabilities | 493,745 | 523,241 |
| Noncurrent liabilities: | | |
| Long-term convertible debt, net | 843,616 | 830,417 |
| Long-term contingent consideration | 48,930 | 46,883 |
| Other long-term liabilities | 97,432 | 58,647 |
| Total liabilities | 1,483,723 | 1,459,188 |
| Stockholders' equity: | | |
| Common stock, \$0.001 par value: 500,000,000 shares authorized; 179,604,381 and 178,252,954 shares issued and outstanding, respectively. | 180 | 178 |
| Additional paid-in capital | 4,782,916 | 4,669,926 |
| Company common stock held by Nonqualified Deferred Compensation Plan (the NQDC) | (9,961) | (13,301) |
| Accumulated other comprehensive income | 37,003 | 5,271 |
| Accumulated deficit | (1,735,733) | (1,694,134) |
| Total stockholders' equity | 3,074,405 | 2,967,940 |
| Total liabilities and stockholders' equity | \$ 4,558,128 | \$ 4,427,128 |

(1) December 31, 2018 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, 2018, filed with the SEC on February 28, 2019.

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

BIOMARIN PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)
Three and Nine Months Ended September 30, 2019 and 2018
(In thousands, except per share amounts)
(unaudited)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|-------------------------------------|--------------------|------------------------------------|--------------------|
| | 2019 | 2018 | 2019 | 2018 |
| REVENUES: | | | | |
| Net product revenues | \$ 450,900 | \$ 386,320 | \$ 1,224,458 | \$ 1,123,205 |
| Royalty and other revenues | 10,197 | 5,386 | 25,147 | 14,793 |
| Total revenues | 461,097 | 391,706 | 1,249,605 | 1,137,998 |
| OPERATING EXPENSES: | | | | |
| Cost of sales | 96,949 | 78,893 | 263,567 | 240,245 |
| Research and development | 172,963 | 161,408 | 542,195 | 520,938 |
| Selling, general and administrative | 170,112 | 148,566 | 493,024 | 440,182 |
| Intangible asset amortization and contingent consideration | 17,063 | 18,580 | 57,114 | 42,009 |
| Gain on sale of intangible assets | — | — | (15,000) | (20,000) |
| Total operating expenses | 457,087 | 407,447 | 1,340,900 | 1,223,374 |
| INCOME (LOSS) FROM OPERATIONS | 4,010 | (15,741) | (91,295) | (85,376) |
| Equity in the loss of BioMarin/Genzyme LLC | (551) | (468) | (780) | (507) |
| Interest income | 5,340 | 6,338 | 17,537 | 17,141 |
| Interest expense | (2,937) | (12,131) | (16,530) | (35,918) |
| Other income, net | 3,960 | 2,589 | 6,038 | 5,266 |
| INCOME (LOSS) BEFORE INCOME TAXES | 9,822 | (19,413) | (85,030) | (99,394) |
| Benefit from income taxes | (45,214) | (6,793) | (46,158) | (25,833) |
| NET INCOME (LOSS) | \$ 55,036 | \$ (12,620) | \$ (38,872) | \$ (73,561) |
| NET INCOME (LOSS) PER SHARE, BASIC | \$ 0.31 | \$ (0.07) | \$ (0.22) | \$ (0.42) |
| NET INCOME (LOSS) PER SHARE, DILUTED | \$ 0.30 | \$ (0.07) | \$ (0.22) | \$ (0.42) |
| Weighted average common shares outstanding, basic | 179,289 | 177,481 | 178,873 | 176,767 |
| Weighted average common shares outstanding, diluted | 185,924 | 177,481 | 178,873 | 176,767 |
| COMPREHENSIVE INCOME (LOSS) | \$ 74,600 | \$ (13,651) | \$ (7,140) | \$ (52,174) |

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

BIOMARIN PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENT OF STOCKHOLDERS' EQUITY
Three and Nine Months Ended September 30, 2019 and 2018
(In thousands)
(unaudited)

| | <u>Three Months Ended September 30,</u> | | <u>Nine Months Ended September 30,</u> | |
|--|---|----------------------------|--|----------------------------|
| | <u>2019</u> | <u>2018</u> | <u>2019</u> | <u>2018</u> |
| Shares of Common Stock | | | | |
| Beginning balance at June 30, 2019 and 2018 and December 31, 2018 and December 31, 2017, respectively ⁽¹⁾ | 179,433 | 177,508 | 178,253 | 175,844 |
| Issuances under equity incentive plans | 171 | 395 | 1,351 | 2,059 |
| Ending balance | <u>179,604</u> | <u>177,903</u> | <u>179,604</u> | <u>177,903</u> |
| Beginning balance of stockholders' equity at June 30, 2019 and 2018 and December 31, 2018 and December 31, 2017, respectively ⁽¹⁾ | \$ 2,960,954 | \$ 2,885,104 | \$ 2,967,940 | \$ 2,808,663 |
| Common stock: | | | | |
| Beginning balance ⁽¹⁾ | 179 | 178 | 178 | 176 |
| Issuances under equity incentive plans, net of tax | 1 | — | 2 | 2 |
| Ending balance | <u>180</u> | <u>178</u> | <u>180</u> | <u>178</u> |
| Additional paid-in capital: | | | | |
| Beginning balance ⁽¹⁾ | 4,744,316 | 4,577,300 | 4,669,926 | 4,483,220 |
| Issuances under equity incentive plans, net of tax | (1,294) | 5,151 | (19,183) | 21,461 |
| Stock-based compensation | 40,144 | 38,366 | 122,212 | 116,136 |
| Common stock held by the NQDC | (250) | — | (692) | — |
| Accounting impact of NQDC Plan change (See Note 9) | — | — | 10,653 | — |
| Ending balance | <u>4,782,916</u> | <u>4,620,817</u> | <u>4,782,916</u> | <u>4,620,817</u> |
| Company common stock held by the NQDC: | | | | |
| Beginning balance ⁽¹⁾ | (10,211) | (13,390) | (13,301) | (14,224) |
| Common stock held by the NQDC | 250 | (25) | 692 | 809 |
| Accounting impact of NQDC Plan change (See Note 9) | — | — | 2,648 | — |
| Ending balance | <u>(9,961)</u> | <u>(13,415)</u> | <u>(9,961)</u> | <u>(13,415)</u> |
| Accumulated other comprehensive income (loss): | | | | |
| Beginning balance ⁽¹⁾ | 17,439 | (1,129) | 5,271 | (22,961) |
| Impact of changes in accounting principle | — | — | — | (586) |
| Other comprehensive income (loss) | 19,564 | (1,031) | 31,732 | 21,387 |
| Ending balance | <u>37,003</u> | <u>(2,160)</u> | <u>37,003</u> | <u>(2,160)</u> |
| Accumulated Deficit: | | | | |
| Beginning balance ⁽¹⁾ | (1,790,769) | (1,677,855) | (1,694,134) | (1,637,548) |
| Impact of changes in accounting principles | — | — | (2,727) | 20,634 |
| Net income (loss) | 55,036 | (12,620) | (38,872) | (73,561) |
| Ending balance | <u>(1,735,733)</u> | <u>(1,690,475)</u> | <u>(1,735,733)</u> | <u>(1,690,475)</u> |
| Total stockholders' equity, ending balances at September 30, 2019 and September 30, 2018, respectively | <u>\$ 3,074,405</u> | <u>\$ 2,914,945</u> | <u>\$ 3,074,405</u> | <u>\$ 2,914,945</u> |

(1) The beginning balances for the nine-month periods were derived from the audited Consolidated Financial Statements included in Company's Annual Report on Form 10-K for the year ended December 31, 2018, filed with the SEC on February 28, 2019.

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, 2019 and 2018
(In thousands)
(unaudited)

| | Nine Months Ended September 30, | |
|--|---------------------------------|----------------|
| | 2019 | 2018 |
| CASH FLOWS FROM OPERATING ACTIVITIES: | | |
| Net loss | \$ (38,872) | \$ (73,561) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Depreciation and amortization | 79,510 | 70,955 |
| Non-cash interest expense | 9,395 | 26,099 |
| (Accretion of discount) Amortization of premium on investments | (1,897) | 769 |
| Stock-based compensation | 121,763 | 112,261 |
| Gain on sale of intangible assets | (15,000) | (20,000) |
| Deferred income taxes | (59,780) | (32,735) |
| Unrealized foreign exchange (gain) loss | 784 | (11,997) |
| Non-cash changes in the fair value of contingent consideration | 5,646 | 12,836 |
| Other | (18) | (459) |
| Changes in operating assets and liabilities: | | |
| Accounts receivable, net | (61,803) | (95,884) |
| Inventory | (52,571) | (10,232) |
| Other current assets | (13,259) | 4,276 |
| Other assets | (7,112) | (3,148) |
| Accounts payable and accrued liabilities | 21,747 | (12,090) |
| Other long-term liabilities | 2,386 | 3,744 |
| Net cash used in operating activities | (9,081) | (29,166) |
| CASH FLOWS FROM INVESTING ACTIVITIES: | | |
| Purchases of property, plant and equipment | (94,241) | (102,541) |
| Maturities and sales of investments | 635,678 | 857,063 |
| Purchases of available-for-sale securities | (528,497) | (439,198) |
| Proceeds from sale of intangible assets | 15,000 | 20,000 |
| Purchase of intangible assets | (8,323) | — |
| Other | (1,747) | (10) |
| Net cash provided by investing activities | 17,870 | 335,314 |
| CASH FLOWS FROM FINANCING ACTIVITIES: | | |
| Proceeds from exercises of awards under equity incentive plans | 21,768 | 55,643 |
| Taxes paid related to net share settlement of equity awards | (40,951) | (34,180) |
| Payment of contingent acquisition consideration | (57,508) | (43,108) |
| Other | (2,025) | — |
| Net cash used in financing activities | (78,716) | (21,645) |
| Effect of exchange rate changes on cash | (835) | (347) |
| NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS | (70,762) | 284,156 |
| Cash and cash equivalents: | | |
| Beginning of period | \$ 493,982 | \$ 598,028 |
| End of period | \$ 423,220 | \$ 882,184 |
| SUPPLEMENTAL CASH FLOW DISCLOSURES: | | |
| Cash paid for income taxes | \$ 6,088 | \$ 15,791 |
| Cash paid for interest | 5,777 | 7,521 |
| SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON-CASH INVESTING AND FINANCING ACTIVITIES: | | |
| Decrease in accounts payable and accrued liabilities related to fixed assets | \$ (5,801) | \$ (7,678) |

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

BIOMARIN PHARMACEUTICAL INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)**(1) NATURE OF OPERATIONS**

BioMarin Pharmaceutical Inc. (the Company) is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare 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 portfolio consists of several commercial therapies and multiple clinical and preclinical product candidates.

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

(2) BASIS OF PRESENTATION

The accompanying Condensed Consolidated Financial Statements have been prepared pursuant to United States (U.S.) generally accepted accounting principles (U.S. GAAP) and the rules and regulations of the SEC for Quarterly Reports on Form 10-Q and do not include all of the information and note disclosures required by U.S. 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, 2018 included in the Company's Annual Report on Form 10-K. The results of operations for the three and nine months ended September 30, 2019 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2019 or any other period.

On January 1, 2019, the Company adopted Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Topic 842, *Leases* (ASC Topic 842) using the modified retrospective method for all lease arrangements at the beginning of the period of adoption. Results for reporting periods beginning January 1, 2019 are presented under ASC Topic 842, while prior period amounts were not adjusted and continue to be presented in accordance with the Company's historical accounting under ASC Topic 840, *Leases*. ASC Topic 842 had a material impact on the Company's Condensed Consolidated Balance Sheet but did not have a significant impact on the Company's consolidated net loss. The Company elected to use the practical expedient allowing the use-of-hindsight and reassessed the lease term for all unexpired leases that commenced before the effective date of ASC Topic 842. For leases that commenced and expired before the effective date of ASC Topic 842, the Company elected not to reassess the expired leases. The Company also elected not to include leases with initial terms of twelve months or less in the recognized right-of-use (ROU) assets and lease liabilities.

As a result of the cumulative impact of adopting ASC Topic 842, the Company recorded lease ROU assets of \$55.9 million and lease liabilities of \$59.0 million as of January 1, 2019, primarily related to real estate and equipment, based on the present value of future lease payments on the date of adoption. The difference between the ROU assets and lease liabilities was recorded as an adjustment to Accumulated Deficit. Refer to Note 11 for additional disclosures required by ASC Topic 842.

On January 1, 2019, the Company adopted Accounting Standards Update No. 2017-12, *Derivatives and Hedging (Topic 815): Targeted Improvements to Accounting for Hedging Activities* (ASU 2017-12), using the modified retrospective method. This ASU provides new guidance about income statement classification and eliminates the requirement to separately measure and report hedge ineffectiveness. Results for reporting periods beginning January 1, 2019 are presented under ASU 2017-12, while prior period amounts were not adjusted and continue to be presented in accordance with the Company's historical accounting. The adoption of this ASU did not have a material impact on the Company's Condensed Consolidated Financial Statements. See Note 10 for additional disclosures required by ASU 2017-12.

U.S. GAAP 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.

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.

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) SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Except as detailed below, there have been no material changes to the Company's significant accounting policies during the nine months ended September 30, 2019, as compared to the significant accounting policies disclosed in Note 3 – *Significant Accounting Policies* included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

Leases

The Company determines if an arrangement is a lease at inception. For leases where the Company is the lessee, ROU assets represent the Company's right to use the underlying asset for the term of the lease and the lease liabilities represent an obligation to make lease payments arising from the lease. ROU assets and lease liabilities are recognized at the lease commencement date based on the present value of the future lease payments over the lease term. The Company uses its incremental borrowing rate based on the information available at the commencement date of the underlying lease arrangement to determine the present value of lease payments. The ROU asset also includes any prepaid lease payments and any lease incentives received. The lease term to calculate the ROU asset and related lease liability includes options to extend or terminate the lease when it is reasonably certain that the Company will exercise the option. The Company's lease agreements generally do not contain any material variable lease payments, residual value guarantees or restrictive covenants.

Lease expense for operating leases is recognized on a straight-line basis over the lease term as an operating expense while expense for financing leases is recognized as depreciation expense and interest expense using the accelerated interest method of recognition. When an arrangement requires payments for lease and non-lease components, the Company has elected to account for lease and non-lease components separately. Lease expense for leases with a term of twelve months or less is recognized on a straight-line basis.

Derivatives and Hedging Activities

The Company accounts for its derivative instruments as either assets or liabilities on the balance sheet and measures them at fair value, which is estimated using current exchange rates and interest rates and takes into consideration the current creditworthiness of the counterparties or the Company, as applicable. For derivatives designated as hedging instruments, the entire change in the fair value of qualifying derivative instruments is recorded in Accumulated Other Comprehensive Income (AOCI) and amounts deferred in AOCI will be reclassified to earnings in the same line item in which the earnings effect of the hedged item is reported. Derivatives not designated as hedging instruments are adjusted to fair value through earnings in Operating Expenses in the Consolidated Statements of Comprehensive Income (Loss).

(4) RECENT ACCOUNTING PRONOUNCEMENTS

Except as described in Note 2 – *Basis of Presentation* and below, there have been no new accounting pronouncements adopted by the Company or new accounting pronouncements issued by the FASB during the nine months ended September 30, 2019, 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, 2018, that the Company believes are of significance or potential significance to the Company.

Accounting Pronouncements Not Yet Adopted

Effective January 1, 2020, the Company will adopt ASU No. 2016-13, *Financial Instruments-Credit Losses: Measurement of Credit Losses on Financial Instruments* (ASU 2016-13), as amended, using a modified retrospective approach, with certain exceptions allowed. The standard amends the guidance for measuring and recording credit losses on financial assets measured at amortized cost by replacing the incurred-loss model with an expected-loss model. This new standard also requires that credit losses related to available-for-sale debt securities be recorded as an allowance through net income rather than by reducing the carrying amount under the current, other-than-temporary-impairment model. The Company is evaluating the impact of the adoption of ASU 2016-13 on its Condensed Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (continued)
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(5) FINANCIAL INSTRUMENTS

All marketable securities were classified as available-for-sale at September 30, 2019 and December 31, 2018.

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of September 30, 2019 and December 31, 2018, respectively:

| As of September 30, 2019 | Amortized Cost | Gross Unrealized Gains | Gross Unrealized Losses | Aggregate Fair Value | Cash and Cash Equivalents | Short-term Marketable Securities ⁽¹⁾ | Long-term Marketable Securities ⁽²⁾ |
|-----------------------------------|---------------------|------------------------------|-------------------------------|-------------------------|------------------------------|---|--|
| Level 1: | | | | | | | |
| Cash | \$ 294,543 | \$ — | \$ — | \$ 294,543 | \$ 294,543 | \$ — | \$ — |
| Level 2: | | | | | | | |
| Money market instruments | 128,678 | — | — | 128,678 | 128,678 | — | — |
| Corporate debt securities | 519,693 | 3,713 | (67) | 523,339 | — | 206,782 | 316,557 |
| U.S. government agency securities | 204,371 | 1,073 | (97) | 205,347 | — | 90,790 | 114,557 |
| Foreign and other | 549 | 142 | (1) | 690 | — | — | 690 |
| Subtotal | 853,291 | 4,928 | (165) | 858,054 | 128,678 | 297,572 | 431,804 |
| Total | <u>\$ 1,147,834</u> | <u>\$ 4,928</u> | <u>\$ (165)</u> | <u>\$ 1,152,596</u> | <u>\$ 423,220</u> | <u>\$ 297,572</u> | <u>\$ 431,804</u> |
| As of December 31, 2018 | Amortized Cost | Gross Unrealized Gains | Gross Unrealized Losses | Aggregate Fair Value | Cash and Cash Equivalents | Short-term Marketable Securities ⁽¹⁾ | Long-term Marketable Securities ⁽²⁾ |
| Level 1: | | | | | | | |
| Cash | \$ 228,809 | \$ — | \$ — | \$ 228,809 | \$ 228,809 | \$ — | \$ — |
| Level 2: | | | | | | | |
| Money market instruments | 205,736 | — | — | 205,736 | 205,736 | — | — |
| Corporate debt securities | 564,852 | 214 | (2,288) | 562,778 | 2,000 | 376,545 | 184,233 |
| Commercial paper | 77,702 | — | — | 77,702 | 21,964 | 55,738 | — |
| U.S. government agency securities | 240,436 | 144 | (697) | 239,883 | 31,474 | 156,967 | 51,442 |
| Foreign and other | 5,126 | 139 | (1) | 5,264 | 3,999 | 1,076 | 189 |
| Subtotal | 1,093,852 | 497 | (2,986) | 1,091,363 | 265,173 | 590,326 | 235,864 |
| Total | <u>\$ 1,322,661</u> | <u>\$ 497</u> | <u>\$ (2,986)</u> | <u>\$ 1,320,172</u> | <u>\$ 493,982</u> | <u>\$ 590,326</u> | <u>\$ 235,864</u> |

(1) The Company's short-term marketable securities mature in one year or less.

(2) The Company's long-term marketable securities mature between one and five years.

As of September 30, 2019, the Company's investments in an unrealized loss position were not significant, and since the Company has the ability and intent to hold all investments that have been in a continuous loss position until maturity or recovery, no other-than-temporary impairment was deemed to have occurred.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)**(6) INTANGIBLE ASSETS**

Intangible assets consisted of the following:

| | September 30, 2019 | December 31, 2018 |
|------------------------------------|-----------------------|----------------------|
| Intangible assets: | | |
| Finite-lived intangible assets | \$ 642,677 | \$ 307,995 |
| Indefinite-lived intangible assets | — | 326,359 |
| Gross intangible assets: | 642,677 | 634,354 |
| Less: Accumulated amortization | (179,828) | (142,546) |
| Net carrying value | <u>\$ 462,849</u> | <u>\$ 491,808</u> |

During the second quarter of 2019, \$326.4 million of indefinite-lived intangible assets were reclassified to definite-lived as the underlying in-process research and development (IPR&D) was placed into service upon receiving European regulatory approval for Palynziq. The Company will straight-line the amortization expense for the underlying IPR&D over its estimated useful life through August 2028.

During the second quarter of 2019, the Company recorded a \$15.0 million gain on sale of intangible assets in the Condensed Consolidated Statements of Comprehensive Income (Loss) due to a third party's achievement of a commercial sales milestone related to a previously sold intangible asset.

(7) PROPERTY, PLANT AND EQUIPMENT

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

| | September 30, 2019 | December 31, 2018 |
|--|-----------------------|----------------------|
| Building and improvements | \$ 709,990 | \$ 694,447 |
| Manufacturing and laboratory equipment | 365,348 | 345,947 |
| Computer hardware and software | 165,235 | 157,787 |
| Leasehold improvements | 52,379 | 41,188 |
| Furniture and equipment | 37,603 | 33,234 |
| Land improvements | 7,239 | 6,551 |
| Land | 83,094 | 77,993 |
| Construction-in-progress | 84,021 | 64,170 |
| | <u>1,504,909</u> | <u>1,421,317</u> |
| Accumulated depreciation | (535,609) | (472,635) |
| Total property, plant and equipment, net | <u>\$ 969,300</u> | <u>\$ 948,682</u> |

The construction-in-progress balance primarily included costs related to significant in-process projects at the Company's facilities in Marin County, California, and Shanbally, Ireland.

Depreciation expense for the three and nine months ended September 30, 2019 was \$24.2 million and \$67.2 million, respectively, of which \$9.8 million and \$24.9 million, respectively, was capitalized into inventory. Depreciation expense for the three and nine months ended September 30, 2018 was \$26.6 million and \$66.7 million, respectively, of which \$8.1 million and \$18.6 million, respectively, was capitalized into inventory.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)**(8) SUPPLEMENTAL BALANCE SHEET INFORMATION**

Inventory consisted of the following:

| | September 30, 2019 | December 31, 2018 |
|-----------------|-----------------------|----------------------|
| Raw materials | \$ 68,391 | \$ 74,616 |
| Work-in-process | 321,073 | 231,064 |
| Finished goods | 219,585 | 225,191 |
| Total inventory | <u>\$ 609,049</u> | <u>\$ 530,871</u> |

Inventory as of September 30, 2019, included manufacturing-related costs for the commercial production of valoctocogene roxaparvec inventory totaling \$7.1 million. Valoctocogene roxaparvec is an investigational gene therapy product candidate for the treatment of severe hemophilia A. The Company must receive marketing approval from the applicable regulators before the valoctocogene roxaparvec inventory can be sold commercially. The Company believes that all material uncertainties related to the ultimate regulatory approval of valoctocogene roxaparvec for commercial sale have been significantly reduced. A number of factors were taken into consideration, including the current status in the drug development process, pivotal clinical trial results for the underlying product candidate, results from meetings with the relevant regulatory authorities prior to the filing of regulatory applications, historical experience, as well as potential impediments to the approval process such as product safety or efficacy, as well as commercialization and marketplace trends. If regulatory approval is not obtained, the manufacturing-related costs for the commercial production of valoctocogene roxaparvec will be expensed.

Accounts Payable and Accrued Liabilities consisted of the following:

| | September 30, 2019 | December 31, 2018 |
|---|-----------------------|----------------------|
| Accounts payable and accrued operating expenses | \$ 226,769 | \$ 207,620 |
| Accrued compensation expense | 132,049 | 149,937 |
| Accrued rebates payable | 55,957 | 43,116 |
| Accrued royalties payable | 27,881 | 19,977 |
| Value added taxes payable | 7,556 | 7,785 |
| Forward foreign currency exchange contracts | 8,731 | 4,178 |
| Lease liability | 10,225 | — |
| Other | 14,577 | 4,677 |
| Total accounts payable and accrued liabilities | <u>\$ 483,745</u> | <u>\$ 437,290</u> |

(9) FAIR VALUE MEASUREMENTS

The Company measures certain financial assets and liabilities at fair value in accordance with the policy described in Note 3 – *Significant Accounting Policies* included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

The following tables present the classification within the fair value hierarchy of financial assets and liabilities not disclosed elsewhere in these Condensed Consolidated Financial Statements that are remeasured on a recurring basis as of September 30, 2019 and December 31, 2018. There were no financial assets or liabilities that were remeasured using a quoted price in active markets for identical assets (Level 1) as of September 30, 2019.

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 September 30, 2019

| | Significant Other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) | Total |
|---------------------------------------|--|--|-----------|
| Assets: | | | |
| Other current assets: | | | |
| NQDC Plan assets | \$ 1,135 | \$ — | \$ 1,135 |
| Other assets: | | | |
| NQDC Plan assets | 15,039 | — | 15,039 |
| Restricted investments ⁽¹⁾ | 3,157 | — | 3,157 |
| Total other assets | 18,196 | — | 18,196 |
| Total assets | \$ 19,331 | \$ — | \$ 19,331 |
| Liabilities: | | | |
| Current liabilities: | | | |
| NQDC Plan liability ⁽²⁾ | \$ 1,135 | \$ — | \$ 1,135 |
| Contingent consideration | — | 10,000 | 10,000 |
| Total current liabilities | 1,135 | 10,000 | 11,135 |
| Other long-term liabilities: | | | |
| NQDC Plan liability ⁽²⁾ | 15,039 | — | 15,039 |
| Contingent consideration | — | 48,930 | 48,930 |
| Total other long-term liabilities | 15,039 | 48,930 | 63,969 |
| Total liabilities | \$ 16,174 | \$ 58,930 | \$ 75,104 |

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, 2018

| | Quoted Price in Active Markets For Identical Assets (Level 1) | Significant Other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) | Total |
|---------------------------------------|---|--|--|------------|
| Assets: | | | | |
| Other current assets: | | | | |
| NQDC Plan assets | \$ — | \$ 370 | \$ — | \$ 370 |
| Restricted investments ⁽¹⁾ | — | 9,581 | — | 9,581 |
| Total other current assets | — | 9,951 | — | 9,951 |
| Other assets: | | | | |
| NQDC Plan assets | — | 12,828 | — | 12,828 |
| Restricted investments ⁽¹⁾ | — | 2,450 | — | 2,450 |
| Strategic investments ⁽³⁾ | 942 | — | — | 942 |
| Total other assets | 942 | 15,278 | — | 16,220 |
| Total assets | \$ 942 | \$ 25,229 | \$ — | \$ 26,171 |
| Liabilities: | | | | |
| Current liabilities: | | | | |
| NQDC Plan liability | \$ 55 | \$ 370 | \$ — | \$ 425 |
| Contingent consideration | — | — | 85,951 | 85,951 |
| Total current liabilities | 55 | 370 | 85,951 | 86,376 |
| Other long-term liabilities: | | | | |
| NQDC Plan liability | 17,598 | 12,828 | — | 30,426 |
| Contingent consideration | — | — | 46,883 | 46,883 |
| Total other long-term liabilities | 17,598 | 12,828 | 46,883 | 77,309 |
| Total liabilities | \$ 17,653 | \$ 13,198 | \$ 132,834 | \$ 163,685 |

- (1) The restricted investments at September 30, 2019 and December 31, 2018 secure the Company's irrevocable standby letters of credit obtained in connection with certain commercial agreements.
- (2) The Company's NQDC Plan was amended during the second quarter of 2019, which resulted in a change to the classification of the obligation associated with the Company's common stock held in the NQDC Plan. The obligation was previously classified as a liability recorded at fair value and has been reclassified into equity and recorded at the shares' respective grant date fair values at June 30, 2019. The change to the NQDC Plan related to the prohibition of participants to diversify investments for deferrals of Company stock contributed into other types of investments. The NQDC Plan liabilities classified as Level 2 represent investments held in plan assets excluding shares of the Company's common stock.
- (3) The Company had investments in marketable equity securities measured using quoted prices in an active market that were considered strategic investments and were included in Other Assets on the Company's Condensed Consolidated Balance Sheets. During the second quarter of 2019, the Company realized an immaterial gain upon the sale of the shares.

There were no transfers between levels during the three and nine months ended September 30, 2019.

BIOMARIN PHARMACEUTICAL INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (continued)
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

The following table represents a roll-forward of contingent consideration.

| | | |
|---|----|---------------|
| Contingent consideration at December 31, 2018 | \$ | 132,834 |
| Changes in fair value of other contingent consideration | | 19,916 |
| Milestone payments to Ares Trading S.A. (Merck Serono) | | (83,472) |
| Milestone payments to former LEAD Therapeutics, Inc. shareholders | | (5,987) |
| Realized gain on settlement of contingent consideration | | (1,928) |
| Foreign exchange remeasurement of Euro denominated contingent consideration | | (2,433) |
| Contingent consideration at September 30, 2019 | \$ | <u>58,930</u> |

(10) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

The Company uses forward foreign currency exchange contracts (forward 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 product revenues and operating expenses being denominated in currencies other than the U.S. Dollar (USD), primarily the Euro. The Company designates certain of these forward contracts as hedging instruments and also uses forward contracts for economic hedging purposes which are not designated as hedging instruments. Whether designated or undesignated, these forward contracts protect against the reduction in value of forecasted foreign currency cash flows resulting from net product revenues, operating expenses and asset or liability positions designated in currencies other than the USD. To receive hedge accounting treatment, derivatives that hedge cash flows must be highly effective at offsetting changes to expected future cash flows on hedged transactions. The Company does not hold or issue derivative instruments for trading or speculative purposes.

The following table summarizes the Company's derivatives designated as hedging instruments outstanding as of September 30, 2019 (notional amounts in millions):

| Foreign Exchange Contracts | Number of Contracts | Aggregate Notional Amount in Foreign Currency | Maturity |
|----------------------------|---------------------|---|----------------------|
| Australian Dollars – Sell | 6 | 2.8 | Oct 2019 - Dec 2019 |
| Canadian Dollars – Sell | 24 | 28.1 | Oct 2019 - Sept 2020 |
| Colombian Pesos – Sell | 12 | 89,400.0 | Oct 2019 - Sept 2020 |
| Euros – Purchase | 150 | 189.4 | Oct 2019 - Sept 2022 |
| Euros – Sell | 453 | 624.2 | Oct 2019 - Sept 2022 |
| Norwegian Krone – Sell | 3 | 11.7 | Oct 2019 - Dec 2019 |
| Total | <u>648</u> | | |

The following table summarizes the Company's derivatives not designated as hedging instruments outstanding as of September 30, 2019 (notional amounts in millions):

| Foreign Exchange Contracts | Number of Contracts | Aggregate Notional Amount in Foreign Currency | Maturity |
|---------------------------------|---------------------|---|---------------------|
| Colombian Pesos – Sell | 2 | 96,000.0 | Oct 2019 - Nov 2019 |
| Euros – Purchase | 2 | 44.1 | Oct 2019 - Nov 2019 |
| Great British Pounds - Purchase | 1 | 4.9 | Oct 2019 |
| Great British Pounds - Sell | 1 | 2.0 | Oct 2019 |
| Rubles – Sell | 2 | 1,420.0 | Oct 2019 - Nov 2019 |
| Total | <u>8</u> | | |

BIOMARIN PHARMACEUTICAL INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (continued)
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

The fair value carrying amounts of the Company's derivatives, as classified within the fair value hierarchy, were as follows:

| Balance Sheet Location | September 30, 2019 | December 31, 2018 |
|---|--------------------|-------------------|
| Derivatives designated as hedging instruments: | | |
| Asset Derivatives - Level 2 ⁽¹⁾ | | |
| Other current assets | \$ 29,900 | \$ 12,686 |
| Other assets | 22,892 | 10,324 |
| Subtotal | \$ 52,792 | \$ 23,010 |
| Liability Derivatives - Level 2 ⁽¹⁾ | | |
| Accounts payable and accrued liabilities | \$ 7,875 | \$ 4,036 |
| Other long-term liabilities | 7,641 | 3,653 |
| Subtotal | \$ 15,516 | \$ 7,689 |
| Derivatives not designated as hedging instruments: | | |
| Asset Derivatives - Level 2 ⁽¹⁾ | | |
| Other current assets | \$ 71 | \$ 168 |
| Other assets | — | — |
| Subtotal | \$ 71 | \$ 168 |
| Liability Derivatives - Level 2 ⁽¹⁾ | | |
| Accounts payable and accrued liabilities | \$ 858 | \$ 142 |
| Other long-term liabilities | — | — |
| Subtotal | \$ 858 | \$ 142 |
| Total Derivatives Assets | \$ 52,863 | \$ 23,178 |
| Total Derivatives Liabilities | \$ 16,374 | \$ 7,831 |

(1) For additional discussion of fair value measurements, see Note 3 – *Summary of Significant Accounting Policies* included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

The following tables summarize the impact of gains and losses from the Company's derivatives on its Condensed Consolidated Financial Statements for the period presented.

| Derivatives Designated as Cash Flow Hedging Instruments | Three Months Ended September 30, 2019 | Nine Months Ended September 30, 2019 |
|--|--|---|
| Amount of Gain (Loss) Recognized in Other Comprehensive Income | \$ 23,973 | \$ 35,431 |

| | Three Months Ended September 30, 2019 | | Nine Months Ended September 30, 2019 | |
|--|--|------------|--|------------|
| Derivatives Designated as Cash Flow Hedging Instruments | Cash Flow Hedging Gains (Losses) Reclassified into Earnings | | Cash Flow Hedging Gains (Losses) Reclassified into Earnings | |
| Net product revenues as reported | \$ 450,900 | \$ 6,196 | \$ 1,224,458 | \$ 11,171 |
| Operating expenses as reported | \$ 457,087 | \$ (1,388) | \$ 1,340,900 | \$ (1,885) |
| Derivatives Not Designated as Hedging Instruments | Gains (Losses) Recognized in Earnings | | Gains (Losses) Recognized in Earnings | |
| Operating Expenses | \$ (1,286) | | \$ (5,182) | |

BIOMARIN PHARMACEUTICAL INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (continued)
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

As of September 30, 2019, the Company expects to reclassify unrealized gains of \$19.2 million from AOCI to earnings as the forecasted revenue and operating expense transactions occur over the next 12 months.

The Company is exposed to counterparty credit risk on its derivatives. The Company has established and maintains strict counterparty credit guidelines and enters into hedging agreements with financial institutions that are investment grade or better to minimize the Company's exposure to potential defaults. The Company is not required to pledge collateral under these agreements.

(11) LEASES

The following table presents the Company's right-of-use (ROU) assets and lease liabilities as of September 30, 2019:

| Lease Classification | Classification | September 30, 2019 |
|-------------------------|--|-----------------------|
| Assets: | | |
| Operating | Other Assets | \$ 51,284 |
| Financing | Other Assets | 10,346 |
| Total ROU assets | | <u>\$ 61,630</u> |
| Liabilities: | | |
| Current: | | |
| Operating | Accounts payable and accrued liabilities | \$ 7,273 |
| Financing | Accounts payable and accrued liabilities | 2,952 |
| Noncurrent: | | |
| Operating | Other long-term liabilities | 45,499 |
| Financing | Other long-term liabilities | 9,544 |
| Total lease liabilities | | <u>\$ 65,268</u> |

Maturities of lease liabilities as of September 30, 2019 by fiscal year were as follows:

| Maturity of Lease Liabilities | Operating | Financing | Total |
|------------------------------------|------------------|------------------|------------------|
| Remainder of 2019 | \$ 3,115 | \$ 3,204 | \$ 6,319 |
| 2020 | 9,925 | 3,464 | 13,389 |
| 2021 | 8,721 | 2,865 | 11,586 |
| 2022 | 8,406 | 2,258 | 10,664 |
| 2023 | 7,504 | 1,748 | 9,252 |
| Thereafter | 27,464 | — | 27,464 |
| Total lease payments | <u>65,135</u> | <u>13,539</u> | <u>78,674</u> |
| Less: Interest | (12,363) | (1,043) | (13,406) |
| Present value of lease liabilities | <u>\$ 52,772</u> | <u>\$ 12,496</u> | <u>\$ 65,268</u> |

| Lease Cost | Classification | Three Months Ended September 30, 2019 | Nine Months Ended September 30, 2019 |
|--------------------------|--------------------|--|---|
| Operating ⁽¹⁾ | Operating Expenses | \$ 3,535 | \$ 9,946 |
| Financing: | | | |
| Amortization | Operating Expenses | 600 | 1,811 |
| Interest expense | Operating Expenses | 142 | 455 |
| Total lease costs | | <u>\$ 4,277</u> | <u>\$ 12,212</u> |

(1) Includes short-term leases and variable lease costs, both of which were not material.

BIOMARIN PHARMACEUTICAL INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (continued)
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

| Other Information | September 30, 2019 |
|---|-------------------------------|
| Weighted average remaining lease term (in years): | |
| Operating leases | 8.0 |
| Financing leases | 4.2 |
| Weighted average discount rate: | |
| Operating leases | 5.2% |
| Financing leases | 5.4% |

As of September 30, 2019, no operating leases are expected to commence in the remainder of 2019.

| Supplemental Cash Flow Information | Nine Months Ended September 30, 2019 | |
|---|---|-------|
| Cash paid for amounts included in the measurement of lease liabilities: | | |
| Cash used in operating activities: | | |
| Operating leases | \$ | 5,918 |
| Financing leases | \$ | 454 |
| Cash used in financing activities: | | |
| Financing leases | \$ | 2,027 |
| ROU assets obtained in exchange for lease obligations: | | |
| Operating leases | \$ | 9,268 |
| Financing leases | \$ | 72 |

Lease Commitments as of December 31, 2018

Minimum lease payments for future years as of December 31, 2018 were as follows:

| | | |
|------------|----|--------|
| 2019 | \$ | 12,976 |
| 2020 | | 12,549 |
| 2021 | | 11,198 |
| 2022 | | 10,574 |
| 2023 | | 9,993 |
| Thereafter | | 27,701 |
| Total | \$ | 84,991 |

Rent expense for the year ended December 31, 2018 was \$12.2 million. Total deferred rent accruals at December 31, 2018 were \$2.1 million, of which \$0.5 million was short-term.

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) DEBT
Convertible Notes

As of September 30, 2019, the Company had outstanding fixed-rate notes with varying maturities for an undiscounted aggregate principal amount of \$870.0 million (collectively the Notes). The Notes are senior subordinated convertible obligations, and interest is payable in arrears, semi-annually. The following table summarizes information regarding the Company's convertible debt:

| | September 30, 2019 | December 31, 2018 |
|--|-----------------------|----------------------|
| 1.50% senior subordinated convertible notes due in October 2020 (the 2020 Notes) | 374,993 | 374,993 |
| Unamortized discount | (15,780) | (26,581) |
| Unamortized deferred offering costs | (1,359) | (2,334) |
| Convertible Notes due in 2020, net | 357,854 | 346,078 |
| 0.599% senior subordinated convertible notes due in August 2024 (the 2024 Notes) | 495,000 | 495,000 |
| Unamortized discount | (6,887) | (7,946) |
| Unamortized deferred offering costs | (2,351) | (2,715) |
| Convertible Notes due in 2024, net | 485,762 | 484,339 |
| Total convertible debt, net | \$ 843,616 | \$ 830,417 |
| Fair value of fixed rate convertible debt | | |
| Convertible Notes due in October 2020 ⁽¹⁾ | 384,713 | 419,722 |
| Convertible Notes due in August 2024 ⁽¹⁾ | 490,297 | 491,626 |
| Total fair value of fixed rate convertible debt | \$ 875,010 | \$ 911,348 |

(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. For additional discussion of fair value measurements, see Note 3 – *Summary of Significant Accounting Policies* included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|-------------------------------------|------------------|------------------------------------|------------------|
| | 2019 | 2018 | 2019 | 2018 |
| Coupon interest expense | \$ 2,271 | \$ 3,331 | \$ 6,681 | \$ 9,819 |
| Amortization of debt issuance costs | 508 | 1,008 | 1,522 | 3,018 |
| Accretion of discount on convertible notes | 4,005 | 7,792 | 11,860 | 23,081 |
| Total interest expense on convertible debt | \$ 6,784 | \$ 12,131 | \$ 20,063 | \$ 35,918 |

See Note 12 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018 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)

Revolving Credit Facility

In October 2018, the Company entered into an unsecured revolving credit facility of up to \$200.0 million (the 2018 Credit Facility). The 2018 Credit Facility includes a letter of credit subfacility and a swingline loan subfacility and is intended to finance ongoing working capital needs and for other general corporate purposes. Borrowings under the 2018 Credit Facility bear interest, at the Company's option, at a rate equal to either (a) the LIBOR rate (except that if LIBOR is less than zero it shall be deemed to be zero for purposes of the 2018 Credit Facility), or LIBOR successor rate, plus an applicable margin ranging from 1.00% to 1.95% per annum, based upon the Company's net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods, or (b) the Base Rate, generally the prime lending rate, plus an applicable margin ranging from 0.00% to 0.95%, based upon the Company's net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods. Commitment fees payable on the undrawn amount range from 0.15% to 0.35% per annum based upon the Company's net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods. The Company's obligations under the Credit Facility are guaranteed by its direct subsidiary, California Corporate Center Acquisition LLC, and such obligations may in the future be guaranteed from time to time by certain other material domestic subsidiaries. The 2018 Credit Facility matures on October 19, 2021 at which time all outstanding amounts become due and payable, except that if at least \$100.0 million aggregate principal amount of the 2020 Notes remain outstanding on August 1, 2020 and certain other conditions have not been met, the Company may be required to repay all amounts borrowed under the 2018 Credit Facility on August 1, 2020. The Company incurred approximately \$1.0 million of issuance costs, which will be amortized to Interest Expense over the term of the 2018 Credit Facility. The 2018 Credit Facility contains financial covenants requiring the Company to maintain a minimum interest coverage ratio and a minimum liquidity requirement. As of September 30, 2019, and December 31, 2018, there were no outstanding amounts due on nor any usage of the 2018 Credit Facility. As of September 30, 2019, the Company and certain of its subsidiaries that served as guarantors were in compliance with all covenants.

(13) ACCUMULATED OTHER COMPREHENSIVE INCOME

The following table summarizes amounts reclassified out of AOCI and their effect on the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2019 and 2018.

| | Three Months Ended September 30, | | Nine Months Ended September 30, | | Condensed Consolidated Statement of Comprehensive Income (Loss) Classification |
|---------------------------------------|-------------------------------------|----------|------------------------------------|------------|--|
| | 2019 | 2018 | 2019 | 2018 | |
| Gains (losses) on cash flow hedges: | | | | | |
| Forward contracts | \$ 6,196 | \$ 5,025 | \$ 11,171 | \$ (6,683) | Net product revenues |
| Forward contracts | (1,388) | 498 | (1,885) | 3,762 | Operating expenses |
| Total gain (loss) on cash flow hedges | \$ 4,808 | \$ 5,523 | \$ 9,286 | \$ (2,921) | |

The following tables summarize changes in the accumulated balances for each component of AOCI, including current period other comprehensive income (loss) and reclassifications out of AOCI for the three and nine months ended September 30, 2019 and 2018.

| | Three Months Ended September 30, 2019 | | | |
|--|---|--|---------|-----------|
| | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) on Available for-Sale Debt Securities | Other | Total |
| AOCI balance at June 30, 2019 | \$ 14,181 | \$ 3,271 | \$ (13) | \$ 17,439 |
| Other comprehensive income (loss) before reclassifications | 23,973 | 522 | (3) | 24,492 |
| Less: net gain (loss) reclassified from AOCI | 4,808 | — | — | 4,808 |
| Tax effect | — | (120) | — | (120) |
| Net current-period other comprehensive income (loss) | 19,165 | 402 | (3) | 19,564 |
| AOCI balance at September 30, 2019 | \$ 33,346 | \$ 3,673 | \$ (16) | \$ 37,003 |

BIOMARIN PHARMACEUTICAL INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (continued)
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

Nine Months Ended September 30, 2019

| | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) on Available for-Sale Debt Securities | Other | Total |
|--|--|--|----------------|------------------|
| AOCI balance at December 31, 2018 | \$ 7,201 | \$ (1,917) | \$ (13) | \$ 5,271 |
| Other comprehensive income (loss) before reclassifications | 35,431 | 7,256 | (3) | 42,684 |
| Less: gain (loss) reclassified from AOCI | 9,286 | — | — | 9,286 |
| Tax effect | — | (1,666) | — | (1,666) |
| Net current-period other comprehensive income (loss) | 26,145 | 5,590 | (3) | 31,732 |
| AOCI balance at September 30, 2019 | <u>\$ 33,346</u> | <u>\$ 3,673</u> | <u>\$ (16)</u> | <u>\$ 37,003</u> |

Three Months Ended September 30, 2018

| | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) on Available for-Sale Debt Securities | Other | Total |
|--|--|--|----------------|-------------------|
| AOCI balance at June 30, 2018 | \$ 2,568 | \$ (3,686) | \$ (11) | \$ (1,129) |
| Other comprehensive income (loss) before reclassifications | 3,355 | 1,475 | — | 4,830 |
| Less: gain (loss) reclassified from AOCI | 5,523 | — | — | 5,523 |
| Tax effect | — | (338) | — | (338) |
| Net current-period other comprehensive income (loss) | (2,168) | 1,137 | — | (1,031) |
| AOCI balance at September 30, 2018 | <u>\$ 400</u> | <u>\$ (2,549)</u> | <u>\$ (11)</u> | <u>\$ (2,160)</u> |

Nine Months Ended September 30, 2018

| | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) on Available for-Sale Debt Securities | Other | Total |
|--|--|--|----------------|-------------------|
| AOCI balance at December 31, 2017 | \$ (20,232) | \$ (2,722) | \$ (7) | \$ (22,961) |
| Impact of change in accounting principle | — | (586) | — | (586) |
| AOCI balance at January 1, 2018 | \$ (20,232) | \$ (3,308) | \$ (7) | \$ (23,547) |
| Other comprehensive income (loss) before reclassifications | 17,711 | 985 | (4) | 18,692 |
| Less: gain (loss) reclassified from AOCI | (2,921) | — | — | (2,921) |
| Tax effect | — | (226) | — | (226) |
| Net current-period other comprehensive income (loss) | 20,632 | 759 | (4) | 21,387 |
| AOCI balance at September 30, 2018 | <u>\$ 400</u> | <u>\$ (2,549)</u> | <u>\$ (11)</u> | <u>\$ (2,160)</u> |

(14) REVENUE, CREDIT CONCENTRATIONS AND GEOGRAPHIC INFORMATION

The Company operates in one business segment, which primarily focuses on the development and commercialization of innovative therapies for people with serious and life-threatening rare diseases and medical conditions. The Company considers there to be revenue concentration risks for regions where Net Product Revenues exceed 10% of consolidated Net Product

BIOMARIN PHARMACEUTICAL INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (continued)
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

Revenues. The concentration of the Company's Net Product Revenues within the regions below may have a material adverse effect on the Company's revenues and results of operations if sales in the respective regions experience difficulties.

The following table disaggregates Total Revenues from external customers and collaborative partners by geographic region. Net product revenues by geographic region are based on patient location for the Company's commercial products, except for Aldurazyme. Although Sanofi Genzyme (Genzyme) sells Aldurazyme worldwide, the revenues earned by the Company are included in the U.S. region, as the transactions are with Genzyme whose headquarters is located in the U.S. Genzyme is the Company's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third parties.

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--------------------------------------|-------------------------------------|-------------------|------------------------------------|---------------------|
| | 2019 | 2018 | 2019 | 2018 |
| Total revenues by geographic region: | | | | |
| United States | \$ 201,874 | \$ 174,722 | \$ 562,217 | \$ 528,082 |
| Europe | 125,485 | 111,069 | 369,585 | 323,940 |
| Latin America | 83,799 | 75,400 | 164,132 | 151,955 |
| Rest of world | 49,939 | 30,515 | 153,671 | 134,021 |
| Total revenues | <u>\$ 461,097</u> | <u>\$ 391,706</u> | <u>\$ 1,249,605</u> | <u>\$ 1,137,998</u> |

The following table disaggregates Net Product Revenues by product.

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|----------------------------------|-------------------------------------|-------------------|------------------------------------|---------------------|
| | 2019 | 2018 | 2019 | 2018 |
| Net product revenues by product: | | | | |
| Aldurazyme | \$ 22,852 | \$ 27,593 | \$ 73,945 | \$ 117,652 |
| Brineura | 19,840 | 9,936 | 46,815 | 27,743 |
| Firdapse | 5,668 | 4,977 | 16,262 | 15,080 |
| Kuvan | 120,524 | 113,254 | 340,771 | 321,414 |
| Naglazyme | 94,408 | 103,089 | 279,462 | 269,171 |
| Palynziq | 24,142 | 4,129 | 55,250 | 4,129 |
| Vimizim | 163,466 | 123,342 | 411,953 | 368,016 |
| Total net product revenues | <u>\$ 450,900</u> | <u>\$ 386,320</u> | <u>\$ 1,224,458</u> | <u>\$ 1,123,205</u> |

The table below disaggregates total Net Product Revenues based on patient location for products sold directly by the Company, and global sales of Aldurazyme, which is marketed by Genzyme, the Company's sole customer for Aldurazyme.

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|-------------------------------------|-------------------|------------------------------------|---------------------|
| | 2019 | 2018 | 2019 | 2018 |
| United States | \$ 175,127 | \$ 146,742 | \$ 480,330 | \$ 409,294 |
| Europe | 121,363 | 107,851 | 358,041 | 315,270 |
| Latin America | 83,799 | 75,400 | 164,132 | 151,955 |
| Rest of world | 47,759 | 28,734 | 148,010 | 129,034 |
| Total net product revenues marketed by the Company | 428,048 | 358,727 | 1,150,513 | 1,005,553 |
| Aldurazyme net product revenues marketed by Genzyme | 22,852 | 27,593 | 73,945 | 117,652 |
| Total net product revenues | <u>\$ 450,900</u> | <u>\$ 386,320</u> | <u>\$ 1,224,458</u> | <u>\$ 1,123,205</u> |

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 total Net Product Revenues attributed to the Company's largest customers for the periods presented.

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--------------|-------------------------------------|------------|------------------------------------|------------|
| | 2019 | 2018 | 2019 | 2018 |
| Customer A | 16% | 16% | 17% | 17% |
| Customer B | 13% | 12% | 12% | 12% |
| Customer C | 10% | 10% | 11% | 10% |
| Customer D | 5% | 7% | 6% | 11% |
| Customer E | 12% | —% | 6% | —% |
| Customer F | —% | 13% | —% | 6% |
| Total | 56% | 58% | 52% | 56% |

On a consolidated basis, three customers accounted for 23%, 14% and 12% of the September 30, 2019 accounts receivable balance, respectively, compared to December 31, 2018, when two customers accounted for 30% and 16% of the accounts receivable balance, respectively. As of September 30, 2019, and December 31, 2018, the accounts receivable balance for Genzyme included \$67.7 million and \$73.9 million, respectively, of unbilled accounts receivable, which become payable to the Company when the product is sold by 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 sells its products in countries that face economic volatility and weakness. Although the Company has historically collected receivables from customers in such 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 in these countries. The Company believes that the allowances for doubtful accounts related to these countries, if any, is adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries.

(15) STOCK-BASED COMPENSATION

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|----------------------------------|------------------|------------------------------------|-------------------|
| | 2019 | 2018 | 2019 | 2018 |
| Cost of sales | \$ 4,093 | \$ 3,974 | \$ 12,629 | \$ 10,360 |
| Research and development | 14,261 | 14,257 | 43,037 | 43,099 |
| Selling, general and administrative | 20,819 | 18,815 | 66,097 | 58,802 |
| Total stock-based compensation expense | \$ 39,173 | \$ 37,046 | \$ 121,763 | \$ 112,261 |

Stock-based compensation of \$5.1 million and \$13.2 million was capitalized into inventory for the three and nine months ended September 30, 2019, respectively, compared to \$5.3 million and \$14.3 million for the three and nine months ended September 30, 2018, respectively. Capitalized stock-based compensation is recognized as cost of sales when the related product is sold.

Equity Awards with Service-Based Vesting Conditions

During the nine months ended September 30, 2019, the Company granted 1,844,308 RSUs with service-based vesting conditions with a weighted-average fair value of \$91.23 per share.

The Company did not grant any options to purchase shares of common stock during the three months ended September 30, 2019. During the nine months ended September 30, 2019, the Company granted 610,250 options to purchase shares of common stock with a weighted-average fair value of \$36.84 per share.

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 assumptions used to estimate the per share fair value of stock options granted during the periods presented were as follows:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--------------------------|-------------------------------------|-------|------------------------------------|--------------|
| | 2019 | 2018 | 2019 | 2018 |
| Expected volatility | N/A | 36.8% | 37.1 – 37.4% | 36.8 – 38.4% |
| Dividend yield | N/A | 0.0% | 0.0% | 0.0% |
| Expected life (in years) | N/A | 5.7 | 4.6 – 5.8 | 4.6 – 5.7 |
| Risk-free interest rate | N/A | 2.8% | 2.2 – 3.0% | 2.3 – 2.8% |

The Company did not issue any new stock purchase rights under the Employee Stock Purchase Plan during the three months ended September 30, 2019. The Company issued 107,473 new stock purchase rights under the Employee Stock Purchase Plan during the nine months ended September 30, 2019.

Restricted Stock Unit Awards with Performance Conditions

In August 2019, the Compensation Committee of the Board of Directors (Board) approved the grant of 44,260 RSUs with performance-based vesting conditions and a grant date fair value of \$81.00 per RSU. This award is contingent upon obtaining regulatory approval for valoctocogene roxaparovec by January 2022 and the awarded RSUs, if any, vest ratably from the time regulatory approval is obtained through January 2022. The Company evaluated the current timeline and plans for submitting the regulatory applications and determined that for accounting purposes attainment of the performance measure is not probable as of September 30, 2019 as the regulatory approval is outside of the Company's control. Therefore, the Company did not record any expense associated with these awards.

In March 2019, the Compensation Committee and Board approved the grant of 99,010 RSUs with performance-based vesting conditions (base RSUs) and a grant date fair value of \$94.53 per RSU. This award is contingent upon the achievement of a 2019 revenue target and the awarded RSUs, if any, vest ratably over a three-year service period. The Company evaluated the 2019 revenue target in the context of its current 2019 revenue forecast, and related confidence level in the forecast, and determined that attainment of the revenue target was probable for accounting purposes commencing in the first quarter of 2019. The number of shares that may be earned range between 0% and 200% of the base RSUs, depending on the percentage of 2019 "managed revenues" (defined as the Company's net product revenues, excluding net revenues attributable to Aldurazyme, and determined using fixed foreign currency exchange rates) achieved against the target managed revenues, with a threshold achievement level of 75% of target and a ceiling achievement level of 125% of target.

Restricted Stock Unit Awards with Market Conditions

In March 2019, the Compensation Committee and Board approved the grant of 99,010 RSUs with market-based vesting conditions (base TSR-RSUs) to certain executives. These base TSR-RSUs, vest, if at all, in full following a three-year service period only if certain total shareholder return (TSR) results relative to the Nasdaq Biotechnology Index comparative companies are achieved. The number of shares that may be earned range between 0% and 200% of the base TSR-RSUs with a ceiling achievement level of 100% of the base TSR-RSUs in the event the Company's absolute TSR multiplier is above the 50th percentile but the Company's TSR multiplier is negative on an absolute basis. The Company utilized a Monte Carlo simulation model to determine the grant date fair value of \$143.92 per base TSR-RSU. Compensation expense for awards with market conditions is recognized over the service period using the straight-line method and is not reversed if the market condition is not met.

BIOMARIN PHARMACEUTICAL INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (continued)
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(16) INCOME TAXES

The Company utilizes an estimated annual effective tax rate for calculating a tax provision under ASC 740-270. That rate is applied to year-to-date ordinary income or loss in order to compute the year-to-date income tax provision. For the three months ended September 30, 2019, the Company recognized a net tax benefit of \$45.2 million primarily attributed to the quarter fluctuations in the mix and timing of our profits and losses on a territorial basis when applying the estimated annual effective tax rate to the year-to-date earnings.

The Company utilizes the asset and liability method of accounting for income taxes. Under this method, deferred taxes are determined based on the difference between the financial statement and tax bases of assets and liabilities using tax rates expected to be in effect in the years in which the differences are expected to reverse. A valuation allowance is recorded to reduce deferred tax assets to the amount that is more likely than not to be realized. The Company's Dutch subsidiary had a full valuation allowance against a deferred tax asset of \$29.6 million at December 31, 2018. Historical earnings, future taxable income and ongoing prudent and feasible tax planning strategies have been considered in assessing the need for the valuation allowance; and based on projected income and other key operating factors, the Company concluded in the second quarter of 2019 that it is more likely than not that the benefit of these deferred tax assets would be realized. As a result, the amount of the valuation allowance related to the deferred tax assets that are expected to be realized was reversed in the second quarter of 2019, resulting in a net tax benefit of \$27.1 million recognized during the nine months ended September 30, 2019.

(17) NET INCOME (LOSS) PER COMMON SHARE

Potentially issuable shares of common stock include shares issuable upon the exercise of outstanding employee stock option awards, common stock issuable under the Company's ESPP, unvested RSUs, common stock held by the Company's NQDC Plan and contingent issuances of common stock related to convertible debt.

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|-------------------------------------|--------------------|------------------------------------|--------------------|
| | 2019 | 2018 | 2019 | 2018 |
| Numerator: | | | | |
| Net Income (Loss), basic | \$ 55,036 | \$ (12,620) | \$ (38,872) | \$ (73,561) |
| Add: Interest on 2024 notes | 937 | — | — | — |
| Net Income (Loss), diluted | <u>\$ 55,973</u> | <u>\$ (12,620)</u> | <u>\$ (38,872)</u> | <u>\$ (73,561)</u> |
| Denominator: | | | | |
| Weighted-average common shares outstanding, basic | 179,289 | 177,481 | 178,873 | 176,767 |
| Effect of dilutive securities: | | | | |
| Options to purchase common stock | 1,560 | — | — | — |
| Common stock issuable under the 2024 notes | 3,970 | — | — | — |
| Unvested RSUs | 669 | — | — | — |
| Common stock potentially issuable for ESPP purchases | 231 | — | — | — |
| Common shares held by the NQDC | 205 | — | — | — |
| Weighted-average common shares outstanding, diluted | <u>185,924</u> | <u>177,481</u> | <u>178,873</u> | <u>176,767</u> |
| Net Income (Loss) per common share, basic | <u>0.31</u> | <u>(0.07)</u> | <u>(0.22)</u> | <u>(0.42)</u> |
| Net Income (Loss) per common share, diluted | <u>0.30</u> | <u>(0.07)</u> | <u>(0.22)</u> | <u>(0.42)</u> |

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

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, | | Nine Months Ended September 30, | |
|--|-------------------------------------|--------|------------------------------------|--------|
| | 2019 | 2018 | 2019 | 2018 |
| Options to purchase common stock | 5,829 | 7,517 | 7,388 | 7,517 |
| Common stock issuable under the 2018 Notes | — | 3,983 | — | 3,983 |
| Common stock issuable under the 2020 Notes | 3,983 | 3,983 | 3,983 | 3,983 |
| Common stock issuable under the 2024 Notes | — | 3,970 | 3,970 | 3,970 |
| Unvested RSUs | 3,360 | 3,411 | 4,029 | 3,411 |
| Common stock potentially issuable for ESPP purchases | 222 | 407 | 453 | 407 |
| Common stock held by the NQDC | — | 208 | 205 | 208 |
| Total number of potentially issuable shares | 13,394 | 23,479 | 20,028 | 23,479 |

The potential effect of the capped call transactions with respect to the 2020 Notes was excluded from the diluted net loss per share as of September 30, 2019 as the Company's closing stock price on September 30, 2019 did not exceed the conversion price of \$94.15 per share. Although the Company's stock price on September 28, 2018 (the last trading day before September 30, 2018) exceeded the conversion price, the potential effect of the capped call transactions and potential shares issuable under the Company's 0.75% senior subordinated convertible notes due in 2018 and the 2020 Notes were excluded from the calculation of diluted loss per share in the three and nine months ended September 30, 2018 as they were anti-dilutive using the if-converted method. There is no similar capped call transaction associated with the 2024 Notes. See Note 12 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018 for additional information related to the Company's convertible debt and capped call transaction.

(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 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.

Contingent Payments

As of September 30, 2019, the Company was subject to contingent payments totaling approximately \$376.9 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, \$65.5 million relates to the acquisition of certain rights and other assets with respect to Kuvan and Palynziq from a third party and \$239.7 million relates to programs that are no longer being developed.

As of September 30, 2019, the Company recorded a total of \$69.1 million of contingent liabilities, of which \$20.2 million was short-term. See Note 9 to these Condensed Consolidated Financial Statements for further information regarding the fair value of the Company's contingent consideration.

Other Commitments

In the normal course of business, the Company enters into various firm purchase commitments primarily related to active pharmaceutical ingredients and certain other inventory-related items. As of September 30, 2019, such commitments and other minimum contractual obligations for clinical and post-marketing services were estimated at approximately \$131.6 million.

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

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 in this Quarterly Report on Form 10-Q. This discussion contains forward-looking statements that involve risks and uncertainties. When reviewing the discussion below, you should keep in mind the substantial risks and uncertainties that could impact our business. In particular, we encourage you to review the risks and uncertainties described in “Risk Factors” in Part II, Item 1A in this Quarterly Report on Form 10-Q. These risks and uncertainties could cause actual results to differ significantly from those projected in forward-looking statements contained in this report or implied by past results and trends. Forward-looking statements are statements that attempt to forecast or anticipate future developments in our business, financial condition or results of operations. See the section titled “Forward-Looking Statements” that appears at the beginning of this Quarterly Report on Form 10-Q. These statements, like all statements in this report, speak only as of the date of this Quarterly Report on Form 10-Q (unless another date is indicated), and, except as required by law, we undertake no obligation to update or revise these statements in light of future developments.

Overview

We are a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare 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 portfolio consists of several commercial products and multiple clinical and preclinical product candidates. A summary of our major commercial products, including key metrics, as of September 30, 2019, is provided below:

| Major Commercial Products | Indication | U.S. Orphan Drug Exclusivity Expiration ⁽¹⁾ | U.S. Biologic Exclusivity Expiration ⁽²⁾ | EU Orphan Drug Exclusivity Expiration ⁽¹⁾ |
|-------------------------------------|------------------------|--|---|--|
| Aldurazyme (laronidase) | MPS I ⁽³⁾ | Expired | Expired | Expired |
| Brineura (cerliponase alfa) | CLN2 ⁽⁴⁾ | 2024 | 2029 | 2027 |
| Kuvan (sapropterin dihydrochloride) | PKU ⁽⁵⁾ | Expired | Not Applicable ⁽⁵⁾ | 2020 ⁽⁵⁾ |
| Naglazyme (galsulfase) | MPS VI ⁽⁶⁾ | Expired | Expired | Expired |
| Palynziq (pegvaliase-pqpz) | PKU ⁽⁷⁾ | 2025 | 2030 | 2029 |
| Vimizim (elosulfase alpha) | MPS IVA ⁽⁸⁾ | 2021 | 2026 | 2024 |

- (1) See “Government Regulation—Orphan Drug Designation” in Part I, Item 1 of our Annual Report on Form 10-K for the year ended December 31, 2018, filed with the SEC on February 28, 2019 (our “Annual Report”) for further discussion
- (2) See “Government Regulation— Healthcare Reform” in Part I, Item 1 of our Annual Report for further discussion
- (3) For the treatment of Mucopolysaccharidosis I (MPS I)
- (4) For the treatment of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2)
- (5) For the treatment of phenylketonuria (PKU). Kuvan, a small molecule therapy, has been granted orphan drug status in the European Union (EU), which together with pediatric exclusivity, confers 12 years of market exclusivity in the EU that expires in 2020.
- (6) For the treatment of Mucopolysaccharidosis VI (MPS VI)
- (7) For the treatment of PKU in adult patients. Palynziq was approved by the U.S. Food and Drug Administration (FDA) in May 2018 and by the European Commission in May 2019.
- (8) For the treatment of Mucopolysaccharidosis IV Type A (MPS IVA)

A summary of our ongoing major development programs, including key metrics as of September 30, 2019, is provided below:

| Major Product Candidates in Development | Target Indication | U.S. Orphan Designation | EU Orphan Designation | Stage |
|---|-----------------------------|-------------------------|-----------------------|------------------|
| Valoctocogene roxaparvovec | Hemophilia A ⁽¹⁾ | Yes | Yes | Clinical Phase 3 |
| Vosoritide | Achondroplasia | Yes | Yes | Clinical Phase 3 |
| BMN 307 | PKU | Not applicable | Not applicable | Preclinical |

- (1) Hemophilia A is also called factor VIII deficiency or classic hemophilia

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

Business Developments

We continued to grow our commercial business and advance our product candidate pipeline over the course of 2019. We believe that the combination of our internal research programs, acquisitions and partnerships will allow us to continue to develop and commercialize innovative therapies for people with serious and life-threatening rare diseases and medical conditions. Below is a summary of key business developments during the nine months ended September 30, 2019:

Product Approval

- **Palynziq** – In May 2019, the European Commission granted marketing authorization for Palynziq at doses of up to 60 mg once daily, to reduce blood Phe concentrations in patients with PKU aged 16 and older, who have inadequate blood Phe control (blood Phe levels greater than 600 micromol/L) despite prior management with available treatment options. EU commercial sales commenced in the third quarter of 2019 and we anticipate meaningful revenue contributions from this region in 2020.

Continued Emphasis on Research and Development

- **BMN 307** - In September 2019, we submitted a clinical trial application with the Medicines and Healthcare Products Regulatory Agency in the United Kingdom for BMN 307, a gene therapy product candidate for the treatment of PKU. Product to support clinical evaluation is being produced at our gene therapy manufacturing facility, where valoctocogene roxaparvovec is currently made, using a commercial-scale manufacturing process to facilitate rapid clinical development.
- **Valoctocogene roxaparvovec** – In July 2019, we announced our plan to submit marketing applications in both the U.S. and Europe in the fourth quarter of 2019 for valoctocogene roxaparvovec based on recent meetings with the FDA and the European Medicines Agency (EMA). These submissions will be based on the updated three-year Phase 1/2 data and the recently completed Phase 3 interim analysis of patients treated with valoctocogene roxaparvovec.

In August 2019 we announced the discontinuation of the valoctocogene roxaparvovec study with the 4e13 vg/kg dose given the overwhelming preference by patients to be treated with the 6e13 vg/kg dose. Enrollment continues in the GENE8-1 Phase 3 open-label study and the 52-week results are anticipated at the end of 2020. Although the trial is open-label, we have instituted a data access plan designed to significantly mirror a blinded trial, such that only a very limited group of medical personnel who are assisting with monitoring and managing the trial have access to any portion of the ongoing results, and then only to the extent necessary to perform the monitoring and management. We plan to review the data generally and conduct a second interim analysis of the GENE8-1 study in the second quarter of 2020 and anticipate sharing results from that data cut shortly thereafter. Until that time, we are not planning to report additional clinical data from the program.

- **BMN 290** - In August 2019 we announced plans to cease the development of BMN 290 program for the treatment of Friedreich's Ataxia program based on progress of other portfolio assets that have demonstrated stronger product profiles.
- **Vosoritide** – In June 2019, the New England Journal of Medicine published the 42-month results from our Phase 2 study with vosoritide in children ages 5 to 14. We expect top line results from the ongoing fully-enrolled global, Phase 3 study by year-end. The vosoritide development program includes four distinct areas of focus to support global approval, including a large contemporaneous natural history study, which is underway.
- **Brineura** – In February 2019, we announced that twenty-three patients in the ongoing open-label extension study treated with Brineura continued to show a reduced rate of decline compared to a natural history cohort of CLN2 disease for three years as measured by the CLN2 Clinical Rating Scale.

Financial Highlights

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

Key components of our results of operations include the following:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|-------------------------------------|----------|------------------------------------|------------|
| | 2019 | 2018 | 2019 | 2018 |
| Total revenues | \$ 461.1 | \$ 391.7 | \$ 1,249.6 | \$ 1,138.0 |
| Cost of sales | 97.0 | 78.9 | 263.6 | 240.2 |
| Research and development (R&D) expense | 173.0 | 161.4 | 542.2 | 520.9 |
| Selling, general and administrative (SG&A) expense | 170.1 | 148.6 | 493.0 | 440.2 |
| Intangible asset amortization and contingent consideration | 17.0 | 18.6 | 57.1 | 42.0 |
| Gain on sale of intangible assets | — | — | (15.0) | (20.0) |
| Net Income/(Loss) | 55.0 | (12.6) | (38.9) | (73.6) |

We recognized Net Income for the three months ended September 30, 2019 as compared to a Net Loss for the three months ended September 30, 2018, primarily due to the following:

- increased gross profit driven by increased sales revenue from products we market;
- increased tax benefit primarily attributed to quarterly fluctuations in the mix and timing of our profits and losses on a territorial basis. See Benefits from Income Taxes below for additional discussion; partially offset by
- increased sales and marketing expense primarily attributed to pre-commercial activities related to valoctocogene roxaparovec and commercial activities in support of the EU commercial launch and continued U.S. expansion of Palynziq;
- increased general and administrative expense primarily attributed to personnel-related costs resulting from increased headcount to support our growth and consulting and professional services expenses; and
- increased R&D expense primarily attributed to pre-clinical activities related to BMN 307 and clinical activities related to our vosoritide and valoctocogene roxaparovec development program partially offset by decreased R&D expense related to Palynziq for which we began capitalizing manufacturing costs upon FDA approval in May 2018, and a decrease in tralesenidase alfa clinical manufacturing costs.

The decrease in Net Loss for the nine months ended September 30, 2019 as compared to the nine months ended September 30, 2018 was primarily attributed to the following:

- increased gross profit driven by increased sales revenue from products we market;
- increased tax benefit primarily attributed to quarterly fluctuations in the mix and timing of our profits and losses on a territorial basis and reversals of certain Dutch tax reserves that were no longer required; partially offset by
- increased sales and marketing expense primarily in support of the EU commercial launch and continued U.S. expansion of Palynziq and valoctocogene roxaparovec pre-commercialization activities;
- increased R&D expense primarily attributed to preclinical and manufacturing activities related to our PKU gene therapy development program and clinical activities related to our valoctocogene roxaparovec development program partially offset by decreased R&D expense related to Palynziq for which we began capitalizing manufacturing costs upon FDA approval in May 2018, and a decrease in tralesenidase alfa clinical manufacturing costs; and
- increased intangible asset amortization related to the Palynziq in-process research and development assets that were placed into service following EU approval in May 2019.

See "Results of Operations" below for additional information related to the Net Income/(Loss) fluctuations presented above.

Our cash, cash equivalents and investments totaled approximately \$1.2 billion as of September 30, 2019, compared to \$1.3 billion as of December 31, 2018. 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, cash equivalents or investments 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.

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

Critical Accounting Policies, Estimates and Judgments

In preparing our Condensed Consolidated Financial Statements in accordance with U.S. generally accepted accounting principles (U.S. GAAP) and pursuant to the rules and regulations promulgated by the Securities and Exchange Commission (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. On an ongoing basis, we evaluate our estimates and discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions.

There have been no significant changes to our critical accounting policies, estimates and judgments during the nine months ended September 30, 2019, compared to the critical accounting policies, estimates and judgments 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, 2018.

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 on our results of operations and financial condition.

Results of Operations

Revenues

Net Product Revenues consisted of the following:

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|----------------------------|-------------------------------------|-----------------|----------------|------------------------------------|-------------------|-----------------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Aldurazyme | \$ 22.8 | \$ 27.6 | \$ (4.8) | \$ 73.9 | \$ 117.7 | \$ (43.8) |
| Brineura | 19.8 | 9.9 | 9.9 | 46.8 | 27.7 | 19.1 |
| Firdapse | 5.7 | 5.0 | 0.7 | 16.3 | 15.1 | 1.2 |
| Kuvan | 120.6 | 113.3 | 7.3 | 340.8 | 321.4 | 19.4 |
| Naglazyme | 94.4 | 103.1 | (8.7) | 279.5 | 269.2 | 10.3 |
| Palynziq | 24.1 | 4.1 | 20.0 | 55.2 | 4.1 | 51.1 |
| Vimizim | 163.5 | 123.3 | 40.2 | 412.0 | 368.0 | 44.0 |
| Total net product revenues | <u>\$ 450.9</u> | <u>\$ 386.3</u> | <u>\$ 64.6</u> | <u>\$ 1,224.5</u> | <u>\$ 1,123.2</u> | <u>\$ 101.3</u> |

Net Product Revenues include revenues generated from our approved products. In the U.S., our commercial products, except for Palynziq and Aldurazyme, are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. Palynziq is distributed in the U.S. through certain certified specialty pharmacies under the Palynziq Risk Evaluation and Mitigation Strategy (REMS) program, and Aldurazyme is marketed world-wide by Sanofi Genzyme (Genzyme). Outside the U.S., our commercial products are sold to authorized distributors or directly to government purchasers or hospitals, which act as the end-users. In certain countries, such as in Latin America, governments place large periodic orders for Naglazyme and Vimizim. The timing of these large government orders can be inconsistent and can create significant quarter to quarter variation in our revenues.

The increase in Net Product Revenues for the three months ended September 30, 2019 as compared to the three months ended September 30, 2018 was primarily attributed to the following:

- Vimizim: the increase was primarily attributed to increased sales volume due to government ordering patterns in the Middle East and Latin America;
- Palynziq: the increase was attributed to a combination of revenue from more patients achieving maintenance dosing and new patients initiating therapy in the U.S. as the product launched in the third quarter of 2018;
- Brineura: the increase was primarily attributed to growth in the number of patients in all regions; and
- Kuvan: the increase was primarily attributed to increased sales volume driven by an increase in the number of patients in the U.S market; partially offset by

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

- Naglazyme: the decrease was primarily attributed to decreased sales volume driven by government ordering patterns from certain Latin American and European countries; and
- Aldurazyme: the decrease was primarily attributed to decreased sales volume due to the timing of customer acceptance for product shipped to Genzyme.

The increase in Net Product Revenues for the nine months ended September 30, 2019 as compared to the nine months ended September 30, 2018 was primarily attributed to the following:

- Palyngiq: the increase was attributed to a combination of revenue from more patients achieving maintenance dosing and new patients initiating therapy in the U.S. as the product launched in the third quarter of 2018;
- Vimizim: the increase was primarily attributed to increased sales volume due to government ordering patterns in the Middle East and Latin America;
- Kuvan: the increase was primarily attributed to increased sales volume driven by an increase in the number of patients in Europe and North America;
- Brineura: the increase was primarily attributed to an increase in the number of patients in Europe;
- Naglazyme: the increase was primarily attributed to increased sales volume driven by government ordering patterns from certain Latin American and European countries; partially offset by
- Aldurazyme: the decrease was primarily attributed to decreased sales volume due to the timing of customer acceptance for product shipped to Genzyme.

We face exposure to movements in foreign currency exchange rates, primarily the Euro. We use foreign currency exchange contracts to hedge a percentage of our foreign currency exposure.

The following table shows our Net Product Revenues denominated in U.S. Dollar (USD) and foreign currencies (as-converted to USD):

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|---|-------------------------------------|-----------------|----------------|------------------------------------|-------------------|-----------------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Sales denominated in USD | \$ 284.6 | \$ 243.3 | \$ 41.3 | \$ 738.6 | \$ 698.6 | \$ 40.0 |
| Sales denominated in foreign currencies | 166.3 | 143.0 | 23.3 | 485.9 | 424.6 | 61.3 |
| Total Net Product Revenues | \$ 450.9 | \$ 386.3 | \$ 64.6 | \$ 1,224.5 | \$ 1,123.2 | \$ 101.3 |

The net impact of foreign currency exchange rates on product sales denominated in currencies other than USD during the three months ended September 30, 2019 was negative by \$6.9 million, compared to a negative impact of \$10.2 million for the three months ended September 30, 2018. The foreign exchange impacts for the three months ended September 30, 2019 and 2018, were driven by fluctuations in the Euro and Brazilian Real. The net impact of foreign currency exchange rates on product sales denominated in currencies other than USD during the nine months ended September 30, 2019 was negative by \$18.6 million, compared to a positive impact of \$1.7 million for the nine months ended September 30, 2018, both of which were driven primarily by the fluctuations in the Euro, partially offset by the Brazilian Real.

Cost of Sales

Cost of Sales includes raw materials, personnel and facility and other costs associated with manufacturing our commercial products. These costs include production materials, production costs at our manufacturing facilities, third-party manufacturing costs, and internal and external final formulation and packaging costs. Cost of Sales also includes royalties payable to third parties based on sales of our products.

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

The following table summarizes our Cost of Sales and product gross margin:

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|----------------------------|-------------------------------------|----------|---------|------------------------------------|------------|----------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Total Net Product Revenues | \$ 450.9 | \$ 386.3 | \$ 64.6 | \$ 1,224.5 | \$ 1,123.2 | \$ 101.3 |
| Cost of Sales | 97.0 | 78.9 | 18.1 | 263.6 | 240.2 | 23.4 |
| Product gross margin | 79% | 80% | (1)% | 79% | 79% | —% |

Our Cost of Sales increased for the three and nine months ended September 30, 2019 compared to the same periods in 2018 due to increased sales volumes. Product gross profits for the three months ended September 30, 2019 compared to the same period in 2018 were lower due primarily to lower unit prices for products sold. Product gross profits for the nine months ended September 30, 2019 compared to the same periods in 2018 remained flat.

Research and Development

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

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 product 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.

We continuously evaluate the recoverability of costs associated with pre-launch or pre-qualification 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 or pre-qualification 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. Starting in the second quarter of 2019, we have capitalized \$7.1 million of manufacturing related costs for the commercial production of valoctocogene roxaparovec through September 30, 2019 as we believe those costs are likely to be recoverable. See Note 8 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our inventory.

R&D expense consisted of the following:

| | Three Months Ended June 30, | | | Nine Months Ended September 30, | | |
|----------------------------|-----------------------------|-----------------|----------------|---------------------------------|-----------------|----------------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Valoctocogene roxaparovec | \$ 40.9 | \$ 35.7 | \$ 5.2 | \$ 144.8 | \$ 107.9 | \$ 36.9 |
| Vosoritide | 25.3 | 19.4 | 5.9 | 85.4 | 59.8 | 25.6 |
| PKU gene therapy (BMN 307) | 31.9 | 5.3 | 26.6 | 73.1 | 10.6 | 62.5 |
| Palynziq | 17.3 | 20.3 | (3.0) | 57.3 | 77.2 | (19.9) |
| Brineura | 9.2 | 10.7 | (1.5) | 30.4 | 34.9 | (4.5) |
| Tralesinidase alfa | 6.8 | 14.3 | (7.5) | 22.6 | 68.6 | (46.0) |
| Other approved products | 14.1 | 16.1 | (2.0) | 48.1 | 51.7 | (3.6) |
| Early stage programs | 21.1 | 16.7 | 4.4 | 57.9 | 49.3 | 8.6 |
| Other | 6.4 | 22.9 | (16.5) | 22.6 | 60.9 | (38.3) |
| Total | <u>\$ 173.0</u> | <u>\$ 161.4</u> | <u>\$ 11.6</u> | <u>\$ 542.2</u> | <u>\$ 520.9</u> | <u>\$ 21.3</u> |

The increase in R&D expense for the three and nine months ended September 30, 2019 as compared to 2018 was primarily attributed to the following:

- an increase in preclinical and manufacturing activities related to our BMN 307 development program, including clinical manufacturing costs;
- an increase in clinical activity related to our late-stage product candidates valoctocogene roxaparovec and vosoritide; partially offset by
- a decrease in other R&D expenses related to development activities that are not allocated to programs; and
- a decrease in tralesinidase alfa clinical manufacturing costs.

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

For the remainder of 2019, we expect our R&D spending to remain flat compared to 2018 levels primarily due to lower development costs related to valoctocogene roxaparvovec and a reduction in costs following the out license of the tralesenidase alfa to a third party in October 2019, offset by increased costs related to BMN 307 and other programs progressing in their development. We expect to continue incurring R&D expense for the foreseeable future due to long-term clinical activities related to post-approval regulatory commitments for our approved products.

Selling, General and Administrative

Sales and Marketing (S&M) expense primarily consisted of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. General and administrative (G&A) expense primarily consisted of corporate support and other administrative expenses, including employee-related expenses.

Selling, General and Administrative (SG&A) expense consisted of the following:

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|----------------------------------|----------------------------------|----------|---------|---------------------------------|----------|---------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Selling & Marketing expense | \$ 93.5 | \$ 81.2 | \$ 12.3 | \$ 264.7 | \$ 238.4 | \$ 26.3 |
| General & Administrative expense | 76.6 | 67.4 | 9.2 | 228.3 | 201.8 | 26.5 |
| Total SG&A expense | \$ 170.1 | \$ 148.6 | \$ 21.5 | \$ 493.0 | \$ 440.2 | \$ 52.8 |

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|--|----------------------------------|---------|---------|---------------------------------|----------|---------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Selling & Marketing expense by product | | | | | | |
| PKU Products | \$ 32.8 | \$ 31.7 | \$ 1.1 | \$ 97.3 | \$ 88.2 | \$ 9.1 |
| MPS Products | 28.5 | 26.5 | 2.0 | 85.2 | 86.3 | (1.1) |
| Brineura | 11.3 | 10.7 | 0.6 | 31.0 | 31.8 | (0.8) |
| Valoctocogene roxaparvovec | 12.6 | 4.7 | 7.9 | 28.7 | 12.0 | 16.7 |
| Other | 8.3 | 7.6 | 0.7 | 22.5 | 20.1 | 2.4 |
| Total Selling & Marketing expense | \$ 93.5 | \$ 81.2 | \$ 12.3 | \$ 264.7 | \$ 238.4 | \$ 26.3 |

The increase in SG&A expense for the three and nine months ended September 30, 2019 as compared to the same periods in 2018 was primarily attributed to the following:

- an increase in S&M expense related to pre-commercialization activities related to valoctocogene roxaparvovec and commercialization activities in support of the continued U.S. expansion and EU commercial launches of Palynziq; partially offset by
- a decrease in commercial activities related to our other approved products as resources were reallocated to supporting the launch of Palynziq; and
- increased G&A expense primarily attributed to increased personnel-related costs resulting from increased headcount to support our growth and other administrative-related costs.

We expect SG&A expense to increase in future periods as a result of the continued commercial launch of Palynziq, pre-commercialization efforts related to valoctocogene roxaparvovec, and the continued international expansion of Vimizim.

Intangible Asset Amortization, Contingent Consideration and Gain on Sale of Intangible Assets

Changes during the periods presented for Intangible Asset Amortization and Contingent Consideration include:

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|--|----------------------------------|---------|-----------|---------------------------------|-----------|---------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Changes in the fair value of contingent consideration | \$ 0.8 | \$ 11.0 | \$ (10.2) | \$ 19.9 | \$ 19.3 | \$ 0.6 |
| Amortization of intangible assets | 16.3 | 7.6 | 8.7 | 37.2 | 22.7 | 14.5 |
| Total intangible asset amortization and contingent consideration | \$ 17.1 | \$ 18.6 | \$ (1.5) | \$ 57.1 | \$ 42.0 | \$ 15.1 |
| Gain on sale of intangible assets | \$ — | \$ — | \$ — | \$ (15.0) | \$ (20.0) | \$ 5.0 |

Fair value of contingent consideration – the changes in the fair value of the contingent consideration for the three and nine months ended September 30, 2019 were attributable to changes in the estimated probability of achieving development

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

milestones, which was primarily related to the continued progress of the Palynziq program that achieved EU marketing approval in May 2019.

Amortization of intangible assets – the increase in the three and nine months ended September 30, 2019 was primarily due to the Palynziq acquired in-process research and development assets that were placed into service following EU marketing approval in May 2019.

Gain on Sale of Intangible Assets – we recognized a gain of \$15.0 million in the nine months ended September 30, 2019 due to a third party's achievement of a milestone during the second quarter of 2019, related to a previously sold intangible asset. See Note 6 to the accompanying Condensed Consolidated Financial Statements for additional information.

Interest Income

We invest our cash equivalents and investments in U.S. government securities and other high credit quality debt securities in order to limit default and market risk. The change during the periods presented for interest income was as follows:

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|-----------------|----------------------------------|--------|----------|---------------------------------|---------|--------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Interest income | \$ 5.3 | \$ 6.3 | \$ (1.0) | \$ 17.5 | \$ 17.1 | \$ 0.4 |

The decrease in interest income for the three months ended September 30, 2019 compared to 2018 was primarily due to a lower investment balance compared to the prior period. The increase in interest income for the nine months ended September 30, 2019 compared to 2018 was primarily due to a higher average interest rate on investments.

Interest Expense

We incur interest expense on our convertible debt. Interest expense for the periods presented consisted of the following:

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|------------------------|----------------------------------|---------|----------|---------------------------------|---------|-----------|
| | 2019 | 2018 | Change | 2019 | 2018 | Change |
| Total interest expense | \$ 2.9 | \$ 12.1 | \$ (9.2) | \$ 16.5 | \$ 35.9 | \$ (19.4) |

The decrease in interest expense on convertible debt for the three and nine months ended September 30, 2019 compared to 2018 was primarily due to the maturity of our 0.75% senior subordinated convertible notes that matured on October 15, 2018.

See Note 12 to the Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2018 for additional information related to our convertible debt.

Benefit from Income Taxes

For the three and nine months ended September 30, 2019, we recognized an income tax benefit of \$45.2 million and \$46.2 million, respectively, compared to the three and nine months ended September 30, 2018 when we recognized an income tax benefit of \$6.8 million and \$25.8 million, respectively. U.S. and foreign tax expense was computed using a forecasted annual effective tax rate for the three and nine months ended September 30, 2019 and 2018. The Tax Cuts and Jobs Act of 2017 (the 2017 Tax Act), which became effective on January 1, 2018, resulted in significant changes to the U.S. corporate income tax system including a federal statutory rate reduction from 35% to 21% and the elimination or reduction of certain domestic deductions and credits. The 2017 Tax Act changed U.S. international taxation from a worldwide basis to a modified territorial system that includes base erosion prevention measures on foreign earnings. This will result in our foreign subsidiaries being subject to U.S. taxation.

The large benefit recognized during the three months ended September 30, 2019 is primarily attributed to the quarter fluctuations in the mix and timing of our profits and losses on a territorial basis when using an estimated annual effective tax rate under ASC 740-270 applied to year-to-date earnings. The income tax benefit for the three and nine months ended September 30, 2019 also consisted of state, federal and foreign current tax expense offset by deferred tax benefits from federal orphan drug and R&D credits, the tax benefit related to stock option exercises during the period and a valuation allowance release of \$27.1 million in the second quarter of 2019 on foreign net operating loss carryforwards that resulted in a net income tax benefit in the nine month period. The income tax benefit for the three and nine months ended September 30, 2018 also consisted of deferred tax benefits from federal orphan drug and R&D credits and the tax benefit related to stock option exercises during that period and was partially offset by state, federal and foreign current income tax expense, which resulted in a net income tax benefit. We recorded a tax benefit of \$4.6 million associated with a measurement-period adjustment in the nine months ending September 30, 2018 related to the remeasurement of deferred taxes as a result of the 2017 Tax Act.

See Note 14 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2018 for additional discussion of our income taxes.

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

Financial Position, Liquidity and Capital Resources

As of September 30, 2019, we had approximately \$1.2 billion in cash, cash equivalents and investments. We expect to fund our operations with our net product revenues from our commercial products, cash, cash equivalents and investments, supplemented as may become necessary by proceeds from equity or debt financings and loans, or collaborative agreements with corporate partners. We may require additional financing to fund the repayment of our convertible debt, future milestone payments and our future operations, including the commercialization of our products and product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. 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 timing and mix of our funding options could change depending on many factors, including how much we elect to spend on our development programs, potential licenses and acquisitions of complementary technologies, products and companies or if we elect to settle all or a portion of our convertible debt in cash.

In managing our liquidity needs in the U.S., we do not rely on unrepatriated earnings as a source of funds and we have not provided for U.S. federal or state income taxes on these undistributed foreign earnings. We do not record U.S. tax expense on the undistributed earnings of our controlled foreign subsidiaries as these earnings are intended to be indefinitely reinvested offshore. As of September 30, 2019, \$206.0 million of our \$1.2 billion balance of cash, cash equivalents, and 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 regarding income taxes, see Note 14 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2018.

We are mindful that conditions in the current macroeconomic environment could affect our ability to achieve our goals. We sell our products in countries that face economic volatility and weakness. Although we have historically collected receivables from customers in such countries, sustained weakness or further deterioration of the local economies and currencies may cause customers in those countries to be unable to pay for our products. We will continue to monitor these conditions and will attempt to adjust our business processes, as appropriate, to mitigate macroeconomic risks to our business.

Our liquidity and capital resources as of September 30, 2019 and December 31, 2018 were as follows:

| | September 30, 2019 | December 31, 2018 | Change |
|--|-----------------------|-------------------|-------------------|
| Cash and cash equivalents | \$ 423.2 | \$ 494.0 | \$ (70.8) |
| Short-term investments | 297.6 | 590.3 | (292.7) |
| Long-term investments | 431.8 | 235.9 | 195.9 |
| Cash, cash equivalents and investments | <u>\$ 1,152.6</u> | <u>\$ 1,320.2</u> | <u>\$ (167.6)</u> |
| Convertible debt | \$ 843.6 | \$ 830.4 | \$ 13.2 |

Our cash flows for the nine months ended September 30, 2019 and 2018 are summarized as follows:

| | 2019 | 2018 | Change |
|--|-------------------|-------------------|-------------------|
| Cash and cash equivalents at the beginning of the period | \$ 494.0 | \$ 598.0 | \$ (104.0) |
| Net cash used in operating activities | (9.1) | (29.2) | 20.1 |
| Net cash provided by investing activities | 17.9 | 335.3 | (317.4) |
| Net cash used in financing activities | (78.7) | (21.6) | (57.1) |
| Foreign exchange impact | (0.8) | (0.3) | (0.5) |
| Cash and cash equivalents at the end of the period | \$ 423.2 | \$ 882.2 | \$ (459.0) |
| Short-term and long-term investments | 729.4 | 766.1 | (36.7) |
| Cash, cash equivalents and investments | <u>\$ 1,152.6</u> | <u>\$ 1,648.3</u> | <u>\$ (495.7)</u> |

Cash Used in Operating Activities

Cash used in operating activities decreased by \$20.1 million to \$9.1 million in the nine months ended September 30, 2019, compared to \$29.2 million in the nine months ended September 30, 2018. The decrease is primarily attributed to the timing of cash receipts from customers, payments to vendors and higher inventory levels.

Cash Provided by Investing Activities

Net cash provided by investing activities decreased by \$317.4 million to \$17.9 million in the nine months ended September 30, 2019, compared to \$335.3 million during the nine months ended September 30, 2018. The decrease in net cash provided by investing activities during the nine months ended September 30, 2019 was primarily attributable to the lower net maturities of available-for-sale debt securities.

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

Cash Used in Financing Activities

Net cash used in financing activities increased by \$57.1 million to \$78.7 million used in the nine months ended September 30, 2019, compared to \$21.6 million during the nine months ended September 30, 2018. The increase in net cash used in financing activities for the nine months ended September 30, 2019 was primarily attributed to a \$33.9 million decrease in proceeds from the exercise of awards under the incentive plans, a \$14.4 million increase in payments of contingent consideration and an increase of \$6.8 million in cash paid for taxes related to the net settlement of equity awards.

Other Information

Our \$870.0 million (undiscounted) of total convertible debt as of September 30, 2019 will impact our liquidity due to the semi-annual cash interest payments. As of September 30, 2019, our indebtedness consisted primarily of the 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes) and our 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes and, together with the 2020 Notes, the Notes), which, if not converted, will be required to be repaid in cash at maturity in 2020 and 2024, respectively. We will need cash not only to pay the ongoing interest due on the Notes during their term, but also to repay the principal amount of the Notes if not converted.

In the event the conditional conversion feature of the 2020 Notes is triggered, holders of the 2020 Notes will be entitled to convert the 2020 Notes at any time during specified periods at their option. In addition, the 2020 Notes will be freely convertible on or after July 15, 2020. We intend to use the remaining balance of the net proceeds we received from the issuance of the 2024 Notes to repay, repurchase or settle in cash some or all of the 2020 Notes. We may elect to settle conversions of the 2020 Notes in cash, in whole or in part, which could further affect our liquidity. While we could seek to obtain additional 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. Even if holders of the 2020 Notes do not elect to convert their 2020 Notes, we could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of such Notes as a current liability rather than long-term liability (for example, when there are 12 months or less remaining until maturity), which would result in a material reduction of our net working capital.

In October 2018, we entered into an unsecured revolving credit facility of \$200.0 million (the 2018 Credit Facility). The 2018 Credit Facility includes a letter of credit subfacility and a swingline loan subfacility and is also intended to finance ongoing working capital needs and for other general corporate purposes. Borrowings under the 2018 Credit Facility bear interest, at our option, at a rate equal to either (a) the LIBOR rate, or LIBOR successor rate, plus an applicable margin ranging from 1.00% to 1.95% per annum, based upon our net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods, or (b) the Base Rate, generally the prime lending rate, plus an applicable margin ranging from 0.00% to 0.95%, based upon our net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods. Our obligations under the Credit Facility are guaranteed by our direct subsidiary, California Corporate Center Acquisition LLC, and such obligations may in the future be guaranteed from time to time by certain other material domestic subsidiaries. Commitment fees payable on the undrawn amount range from 0.15% to 0.35% per annum based upon our net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods. The 2018 Credit Facility matures on October 19, 2021 at which time all outstanding amounts become due and payable, except that if at least \$100.0 million aggregate principal amount of the 2020 Notes remain outstanding on August 1, 2020 and certain other conditions have not been met, we may be required to repay all amounts borrowed under the 2018 Credit Facility on August 1, 2020. The 2018 Credit Facility contains financial covenants requiring us to maintain a minimum interest coverage ratio and a minimum liquidity requirement. As of September 30, 2019, there were no outstanding amounts due on nor any usage of the 2018 Credit Facility.

For additional discussion about our debt, see Note 12 included in our Annual Report on Form 10-K for the year ended December 31, 2018.

Funding Commitments

We cannot estimate with certainty the cost to complete any of our product development programs. Additionally, 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 product candidates, or if approval of our product candidates is delayed, we will be unable to generate revenue from the sale of these product candidates, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will increase;*
- *If we are unable to successfully develop and maintain manufacturing processes for our 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;*

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

- *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. The R&D expenses of our major development programs from inception to September 30, 2019 were as follows:

| | Since Program Inception |
|----------------------------|------------------------------------|
| Palynziq | \$ 675.0 |
| Valoctocogene roxaparvovec | 545.8 |
| Vosoritide | 404.4 |
| Brineura | 317.5 |
| PKU gene therapy | 91.2 |
| Other approved products | 1,098.5 |

We may need or elect to increase our spending above our current long-term plans to be able to achieve our long-term goals. This may increase our capital requirements, including: costs associated with the commercialization of our products; additional clinical trials; investments in the manufacturing of our commercial products; 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:

- our ability to successfully market and sell our products;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the progress and success of our preclinical studies and clinical trials (including the manufacture of materials for use in such studies and trials);
- 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; and
- the progress of research programs carried out by us.

Off-Balance Sheet Arrangements

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

Contractual and Commercial Obligations

We have contractual and commercial obligations under our convertible debt, leases and other obligations related to R&D activities, purchase commitments, licenses and sales royalties with annual minimums.

Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)
(In millions, except as otherwise disclosed)

Our contractual obligations as of September 30, 2019 are presented in the table below.

| | Payments Due Within | | | | Total |
|---------------------------------|----------------------|-----------------|-----------------|----------------|-------------------|
| | Remainder of 2019 | 2020 - 2021 | 2022 - 2024 | Thereafter | |
| 2020 Notes and related interest | \$ 2.8 | \$ 380.6 | \$ — | \$ — | \$ 383.4 |
| 2024 Notes and related interest | — | 5.9 | 503.9 | — | 509.8 |
| Leases | 6.3 | 25.0 | 25.7 | 21.7 | 78.7 |
| R&D and purchase commitments | 107.2 | 24.4 | — | — | 131.6 |
| Total | <u>\$ 116.3</u> | <u>\$ 435.9</u> | <u>\$ 529.6</u> | <u>\$ 21.7</u> | <u>\$ 1,103.5</u> |

We are also subject to contingent payments related to certain development and regulatory activities and commercial sales and licensing milestones totaling approximately \$376.9 million as of September 30, 2019, which are due upon achievement of certain development and commercial milestones and if they occur before certain dates in the future. Of this amount, \$65.5 million relates to remaining amounts due to a third party from whom we acquired certain rights and other assets with respect to Kuvan and Palynziq in 2016, and \$239.7 million relates to programs that are no longer being developed.

As of September 30, 2019, we recorded \$69.1 million of total contingent liabilities on our Condensed Consolidated Balance Sheets, of which \$20.2 million was short-term.

See Note 18 to our accompanying Condensed Consolidated Financial Statements for additional discussion on our commitments.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Our market risks during the nine months ended September 30, 2019 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, 2018.

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, 2019.

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 Control 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.

None.

Item 1A. Risk Factors

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

We have marked with an asterisk (*) those risk factors below that include a substantive change from or update to the risk factors included in our Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on February 28, 2019.

Risks Related to Our Business

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

We must obtain and maintain regulatory approval to market and sell our product candidates. For example, in the U.S., we must obtain Food and Drug Administration (FDA) approval for each product candidate that we intend to commercialize, and in Europe we must obtain approval from the European Medicines Agency (EMA). The FDA and EMA approval processes are typically lengthy and expensive, and approval is never certain. Accordingly, there are no assurances that we will obtain regulatory approval for any of our product candidates. Furthermore, there can be no assurance that approval of one of our product candidates by one regulatory agency will mean that other agencies will also approve the same product candidate. Similarly, regulatory authorities may approve a product candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates.

We have had fewer interactions with regulatory authorities outside the U.S. and the EU as compared to our interactions with the FDA and EMA. The approval procedures vary among countries and can involve additional clinical testing, and the time required to obtain approval may differ from that required to obtain FDA or EMA approval. Moreover, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Approval by the FDA or EMA does not ensure approval by regulatory authorities in other countries, and approval by one or more foreign regulatory authorities does not ensure approval by regulatory authorities in other foreign countries or by the FDA or EMA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. The foreign regulatory approval process may include all of the risks associated with obtaining FDA or EMA approval. We may not obtain foreign regulatory approvals on a timely basis, if at all. We may not be able to file for regulatory approvals and even if we file we may not receive necessary approvals to commercialize our product candidates in any market.

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 product candidates. 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 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 are not well-established areas, 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 product candidates, we engage in discussions with the FDA and comparable international regulatory authorities regarding our development programs, including discussions about the regulatory requirements for approval. As part of these discussions, we sometimes seek advice in the design of our clinical programs from various regulatory agencies globally, but we do not always follow such guidance. This increases the chance of adverse regulatory actions, but we try to always provide appropriate scientific evidence to support approval. For example, although we designed our Phase 3 study of vosoritide in a manner that we believe can demonstrate efficacy and safety of the product candidate for the target patient population, the FDA may ultimately disagree. Moreover, sometimes different regulatory agencies provide different or conflicting advice. While we attempt to harmonize the advice we receive from multiple regulatory authorities, it is not always practical to do so. Also, we may choose not to harmonize conflicting advice when harmonization would significantly delay clinical trial data or is otherwise inappropriate. 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 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.**

Aldurazyme, Brineura, Kuvan, Naglazyme and Vimizim have received regulatory approval to be commercially marketed and sold in the U.S., the EU and certain other countries, Palynziq has received regulatory approval to be commercially marketed in the U.S. and the EU and Firdapse has received regulatory approval to be commercially marketed 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, import and export requirements and record keeping.

An example of the ongoing regulatory requirements our products are subject to is the Palynziq Risk Evaluation and Mitigation Strategy (REMS) program. In the U.S., Palynziq is only available through the REMS program, which is required by the FDA to mitigate the risk of anaphylaxis while using the product. Notable requirements of our REMS program include the following:

- prescribers must be certified by enrolling in the REMS program and completing training;
- prescribers must prescribe auto-injectable epinephrine with Palynziq;
- pharmacies must be certified with the REMS program and must dispense Palynziq only to patients who are authorized to receive it;
- patients must enroll in the REMS program and be educated about the risk of anaphylaxis by a certified prescriber to ensure they understand the risks and benefits of treatment with Palynziq; and
- patients must have auto-injectable epinephrine available at all times while taking Palynziq.

Failure of prescribers, pharmacies or patients to enroll in our REMS program or to successfully complete and comply with its requirements may result in regulatory action from the FDA or decreased sales of Palynziq. The restrictions and requirements under our REMS program, as well as potential changes to these restrictions and requirements in the future, subject us to increased risks and uncertainties, any of which could harm our business. The requirement for a REMS program can materially affect the potential market for and profitability of a drug. We cannot predict whether the FDA will request, seek to require or ultimately require modifications to, or impose additional requirements on, the Palynziq REMS program, or whether the FDA will permit modifications to the Palynziq REMS program that we consider warranted. Any modifications required or rejected by the FDA could make it more difficult or expensive for us to distribute Palynziq in the U.S., impair the safety profile of Palynziq, disrupt continuity of care for Palynziq patients and/or negatively affect sales of Palynziq.

Moreover, 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. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that

is found to have improperly promoted off-label uses may be subject to significant civil, criminal and administrative penalties. Thus, we will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, the FDA often requires post-marketing testing and surveillance to monitor the effects of 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 of our 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, our value 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 available if a sponsor can establish: that the medicine is intended for the diagnosis, prevention or treatment of (1) a life-threatening or chronically debilitating condition affecting no more than five in 10,000 people in the EU, which is equivalent to around 250,000 people or fewer or (2) a life-threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives it is unlikely that the marketing of the medicinal product in the EU would generate sufficient return to justify the necessary investment. For either of these conditions, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the medicinal product will be of significant benefit to those affected by that condition. 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. In addition, the FDA may approve another drug during a period of orphan drug exclusivity if the second drug is found to be clinically superior to the first drug. In the EU, a ten-year period of market exclusivity (extendable to twelve years for medicines that have complied with an agreed pediatric investigation plan pursuant to Regulation 1901/2006) is available. Orphan drug marketing exclusivity may be lost in the EU if a manufacturer is unable to supply sufficient quantities and marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this medicinal product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. The period of market exclusivity may, in addition, be reduced to six years if, at the end of the fifth year, it can be demonstrated on the basis of available evidence that the criteria for its designation as an orphan medicine are no longer satisfied, for example if the original orphan medicinal product has become sufficiently profitable not to justify maintenance of market exclusivity. Because the extent and scope of patent protection for some of our products is limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible products, 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 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. Moreover, with respect to biologics and gene therapy, it is uncertain how similarity between product candidates designed to treat the same rare disease or condition may affect such product candidates' orphan drug exclusivities. 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 Aldurazyme, Brineura, Naglazyme, Palynziq 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 BLA and approval by the FDA prior to being marketed in the U.S. 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. A similar abridged marketing authorization process is available to biosimilar products in the EU. 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. The BPCIA establishes a period of 12 years of exclusivity for reference products. In Europe, a medicinal product containing a new active substance benefits from eight years of data exclusivity, during which biosimilar applications referring to the data of that product may not be accepted by the regulatory authorities, and a further two years of market exclusivity, during which such biosimilar products may not be placed on the market. The two-year period may be extended to three years if during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved. Our products approved under BLAs in the U.S. or MAAs in Europe, as well as products in development that may be approved under those regimes 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. Likewise, preliminary, initial or interim data from clinical trials should be considered carefully and with caution since the final data may be materially different from the preliminary, initial or interim data, particularly as more patient data become available.**

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. The number of preclinical studies and clinical trials that regulatory authorities require varies 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 product 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 product candidates 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. From time to time, we have and may in the future publish or report preliminary, initial or interim data from our clinical trials, such as the data we have announced from the GENE-8-1 study for valoctocogene roxaparvovec. Preliminary, initial or interim data from our clinical trials may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. In this regard, such data may show initial evidence of clinical benefit, but as patients continue to be followed and more patient data become available, there is a risk that any therapeutic effects will not be durable in patients and/or will decrease over time or cease entirely. Preliminary, initial or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from such preliminary, initial or interim data. As a result, preliminary, initial or interim data should be considered carefully and with caution until the final data are available.

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. Also, as noted above, we do not always follow the advice of regulatory authorities or comply with all of their requests regarding the design of our clinical programs. In those cases, we may choose a development program that is inconsistent with the advice of regulatory authorities, which may limit the jurisdictions

where we conduct clinical trials and/or adversely affect our ability to obtain approval in those jurisdictions where we do not follow the regulatory advice.

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

- slow or insufficient patient enrollment;
- slow recruitment of, and completion of necessary institutional approvals at, clinical sites;
- 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;
- availability of competitive therapies to treat the same indication as our product candidates;
- regulatory requests for additional clinical trials or preclinical 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 valoctocogene roxaparvovec 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 addition to the risks set forth in this Risk Factors section associated with developing and commercializing more traditional pharmaceutical drugs, there are additional, unique risks associated with gene therapy products like our product candidate valoctocogene roxaparvovec (formerly referred to as BMN 270). 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 little or too much of the desired protein or RNA. Although a one-time administration of a gene therapy product like our product candidate valoctocogene roxaparvovec is intended to correct an inborn genetic defect for the entire lifetime of a patient, there is a risk that the therapeutic effect will not be durable and production of the desired protein or RNA will decrease over time or cease entirely. Because the treatment is irreversible, there may be challenges in managing side effects, particularly those caused by potential overproduction of the desired protein. 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. Although numerous companies are currently advancing gene therapy product candidates through clinical trials and the FDA has approved several cell-based gene therapy treatments to date, the FDA has only approved a very small number of vector-based gene therapy products thus far. Moreover, there are very few approved gene therapy products outside the U.S. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for valoctocogene roxaparvovec in any jurisdiction. Regulatory requirements governing gene and cell therapy products are still evolving 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 valoctocogene roxaparvovec 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.

We plan to submit a BLA for valoctocogene roxaparvovec to the FDA in the fourth quarter of 2019. If original FDA approval for valoctocogene roxaparvovec is granted via the accelerated approval pathway, we may be required to conduct a post-marketing confirmatory trial to verify and describe the clinical benefit in support of full approval. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of the FDA's marketing approval for valoctocogene roxaparvovec, which could have a negative effect on our business and financial condition.

Even if we obtain and maintain regulatory approval for valoctocogene roxaparvovec, we may experience delays, and increased costs, in developing, optimizing and operating 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. We invested a considerable amount of capital building our own commercial gene therapy manufacturing facility, which may be subject to significant impairment if our gene therapy programs are unsuccessful. As we develop, seek to optimize and operate the valoctocogene roxaparvovec manufacturing process, we will likely face technical and scientific challenges, considerable capital costs, and potential difficulty in recruiting and hiring experienced, qualified personnel. There may also be unexpected technical or operational issues during clinical or commercial manufacturing campaigns. As a result, we could experience manufacturing delays that prevent us from completing our clinical studies or commercializing valoctocogene roxaparvovec in a timely, or on a profitable, basis, if at all.

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

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 valoctocogene roxaparvovec, the commercial success of valoctocogene roxaparvovec will depend, in part, on the acceptance of physicians, patients and third-party payers 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 valoctocogene roxaparvovec displays a favorable efficacy and safety profile in clinical trials and is ultimately approved, market acceptance of valoctocogene roxaparvovec will not be fully known until after it is launched. Negative public opinion or more restrictive government regulations could have a negative effect on our business and financial condition and may delay or impair the development and commercialization of, and demand for, valoctocogene roxaparvovec.

***We have implemented a data access plan for valoctocogene roxaparvovec, which restricts our management's review of emerging data from these trials. Without access to ongoing data, management does not have the ability to adjust the trials based on such emerging data, which could adversely impact the ultimate outcome of these trials.**

In order to preserve the scientific integrity of the valoctocogene roxaparvovec trials and to allow us to only report on data at intervals that we believe will be meaningful to investors, we have implemented a data access plan related to the ongoing open label trials, which is designed to significantly mirror blinded trials. Pursuant to this plan, the ongoing emerging data are generally not collected by us, with the exception that certain specific data points are collected and reviewed by a small group of medical personnel monitoring and managing the trial, and then, only to the extent necessary to allow them to perform their monitoring responsibilities. As we disclose and publicly discuss prior data from these trials, such discussions do not incorporate any of the currently emerging data that are being collected and reviewed by personnel monitoring the trial and, accordingly, this prior data may differ significantly from more recent data that are only available to such personnel. Further, since our management does not have access to any of the ongoing data and does not have the ability to adjust the trials based on such emerging data, the data access plan could adversely impact the ultimate outcome of the trials.

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. Our future profitability and cash flows depend on our marketing and selling of our products, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any products, 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, 2019, we had cash, cash equivalents and investments totaling \$1.2 billion and long-term debt obligations of \$870.0 million (undiscounted), which consisted of our 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes) and our 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes and, together with the 2020 Notes, the Notes), which, if not converted, will be required to be repaid in cash at maturity in 2020 and 2024, respectively. We will

need cash not only to pay the ongoing interest due on the Notes during their term, but also to repay the principal amount of the Notes if not converted.

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 Palynziq, we are obligated to make certain payments to Merck Serono if sales and development milestones are achieved. The remaining milestone payments that may become payable include up to a maximum of €60 million, in cash, if future sales milestones are met with respect to Kuvan and Palynziq.

We may require additional financing to fund the repayment of our Notes, future milestone payments and our future operations, including the commercialization of our products and 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 our products;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities 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 progress of research programs carried out by us;
- our possible achievement of development and commercial milestones under agreements with third parties, such as the Kuvan and Palynziq milestones under the termination agreements with Merck Serono;
- 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;
- Sanofi Genzyme's (Genzyme) ability to continue to successfully commercialize Aldurazyme; 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 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, 2019, we had \$870.0 million (undiscounted) principal amount of indebtedness, including \$375.0 million (undiscounted) principal amount of indebtedness under the 2020 Notes and \$495.0 million (undiscounted) principal amount of indebtedness under the 2024 Notes. In October 2018, we also entered into an unsecured credit agreement (the 2018 Credit Facility) with Bank of America, N.A., as the administrative agent, swingline lender and a lender, Citibank N.A. as letter of credit issuer and each of Merrill Lynch, Pierce, Fenner & Smith Incorporated, Citibank, N.A. and Wells Fargo Securities, LLC as joint lead arrangers and joint bookrunners, providing up to \$200.0 million in revolving loan commitments and terminated the credit facility that we entered into in November 2016, which had provided for up to \$100.0 million in revolving loans (the 2016 Credit Facility). The 2018 Credit Facility replaced the 2016 Credit Facility. 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, the 2018 Credit Facility contains, and any future indebtedness that we may incur may contain, financial and other restrictive covenants that limit our ability to operate our business, raise capital or make payments under our other indebtedness. If we fail to comply with these covenants or to make payments under our indebtedness when due, then we would be in default under that indebtedness, which could, in turn, result in that and our other indebtedness becoming immediately payable in full. If we default under the 2018 Credit Facility, the outstanding borrowings thereunder could become immediately due and payable, the 2018 Credit Facility lenders could refuse to permit additional borrowings under the facility, or it could lead to defaults under agreements governing our current or future indebtedness, including the indentures governing our Notes. If we default under any of the Notes, such Notes could become immediately due and payable and it could lead to defaults under the other Notes and/or the 2018 Credit Facility.

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

Our outstanding indebtedness consists primarily of the 2020 Notes and 2024 Notes, which, if not converted, will be required to be repaid in cash at maturity in 2020 and 2024, respectively. In addition, in the event the conditional conversion feature of the 2020 Notes is triggered, holders of the 2020 Notes will be entitled to convert the 2020 Notes at any time during specified periods at their option, and the 2020 Notes will be freely convertible on or after July 15, 2020. We may elect to settle conversions of the 2020 Notes in cash, in whole or in part, which could further affect our liquidity. While we could seek to obtain additional 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.

We could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of the 2020 Notes as a current rather than long-term liability (for example, if there are 12 months or less remaining until maturity), which would result in a material reduction of our net working capital. 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. In addition, we also may borrow up to \$200.0 million in revolving loans under the 2018 Credit Facility, which would be required to be repaid in cash at maturity on October 19, 2021, except that if at least \$100.0 million aggregate principal amount of the 2020 Notes remains outstanding on August 1, 2020 and certain other conditions have not been met, we may be required to repay all amounts borrowed under the 2018 Credit Facility on August 1, 2020.

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 scheduled and unannounced inspection by the FDA and international regulatory authorities, before and after product approval, to monitor and ensure compliance with cGMP and other regulations. Our manufacturing facility in the U.S. has been approved by the FDA for the manufacture of Palynziq, and it has been approved by the FDA, the European Commission (EC), and health agencies in other countries for the manufacture of Aldurazyme, Brineura, 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, and it has been approved by the FDA and the EMA as a formulated bulk drug substance manufacturing and quality control facility for Brineura. In addition, our third-party manufacturers' facilities involved with the manufacture of our products 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 our products 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 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 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 (including moving manufacturing from one of our facilities to another one of our facilities or a third-party facility, or from a third-party facility to one of our facilities) may require us to complete clinical trials to receive regulatory approval of any manufacturing modifications.

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 Aldurazyme, Brineura, Naglazyme, Palynziq 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.

We have entered into contractual relationships with third-party manufacturers to produce active ingredients in Firdapse, Kuvan and Palynziq. If those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for Firdapse, Kuvan and Palynziq, 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 Firdapse, Kuvan and Palynziq. We also currently rely on third parties for portions of the manufacture of Aldurazyme, Brineura, Naglazyme, Palynziq 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.

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 and adversely affect our financial results and financial condition.

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 Brineura, 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, 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 products by third-party payers, the sales of our products would be adversely affected or there may be no commercially viable markets for our products.

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

Third-party payers, such as government or private healthcare insurers, carefully review and increasingly challenge the prices charged for drugs. Reimbursement rates from private companies vary depending on the third-party payer, the insurance plan and other factors. Obtaining coverage and adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. 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 payers 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 payers 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 payers in the U.S. Therefore, coverage and reimbursement for drug products can differ significantly from payer to payer. 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 payer 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 will exceed 12 months. Even after a price is negotiated, countries frequently request or require reductions 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, and we expect a significant portion of our international sales of Brineura will also be through such programs. 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 and may continue to 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 in certain jurisdictions. 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, which may prevent us from marketing our product entirely) 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 in all the markets in which we sell our products. The escalating cost of healthcare has led to increased pressure on the healthcare 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 payers have proposed healthcare reforms and cost reductions. A number of federal and state proposals to control the cost of healthcare, 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 and enacted legislation 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. Further, Congress and the executive branch have each indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding healthcare may affect coverage and reimbursement for medical treatment by third-party payers, which may render our products not commercially viable or may adversely affect our future revenues and gross margins.

International operations are also generally subject to extensive price and market regulations, and there are many proposals for additional cost-containment measures, including proposals that would directly or indirectly impose additional price controls or mandatory price cuts or reduce the value of our intellectual property portfolio. As part of these cost containment measures, some countries have imposed and continue to propose revenue caps limiting the annual volume of sales of our products. Some of these caps are significantly below the actual demand in certain countries, and if the trend regarding revenue caps continues, 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 product 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 healthcare reform could increase our costs and 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. In the U.S., the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (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. Since its enactment, there have been judicial and Congressional challenges to certain aspects of the PPACA, as well as recent efforts by the U.S. Presidential administration to repeal or replace certain aspects of the PPACA, and we expect there will be additional challenges and amendments to the PPACA in the future. Since January 2017, the U.S. President has signed two Executive Orders and other directives designed to delay, circumvent, or loosen certain requirements mandated by the PPACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the PPACA. While Congress has not passed legislation repealing the PPACA in its entirety, it has enacted laws that modify certain provisions of the PPACA such as removing penalties, starting January 1, 2019, for not complying with the PPACA's individual mandate to carry health insurance, delaying the implementation of certain ACA-mandated fees, and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. Additionally, on December 14, 2018, a Texas U.S. District Court Judge ruled

that the PPACA is unconstitutional in its entirety because the individual mandate was repealed by Congress as part of the Tax Cuts & Jobs Act. While the Texas U.S. District Court Judge, as well as the current U.S. Presidential administration and the Centers for Medicare and Medicaid Services (CMS), have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the PPACA will impact the PPACA and our business. In addition, other legislative changes have been adopted since the PPACA was enacted. Some of these changes have resulted 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 in the U.S. or abroad, may result in more rigorous coverage criteria and an additional downward pressure on the reimbursement our customers may receive for our products. Recently there has been heightened governmental scrutiny in countries worldwide over the manner in which manufacturers set prices for their marketed products.

In the U.S., there have been several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drug products. Moreover, the U.S. Presidential administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. Although a number of these, and other potential measures may require authorization to become effective, Congress and the U.S. Presidential administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. In addition, individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Moreover, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

Likewise, in many EU countries, legislators and other policymakers continue to propose and implement healthcare cost-containing measures in response to the increased attention being paid to healthcare costs in the EU. Certain of these changes could impose limitations on the prices we will be able to charge for our products and any approved product candidates or the amounts of reimbursement available for these products from governmental and private third-party payers, may increase the tax obligations on pharmaceutical companies or may facilitate the introduction of generic competition with respect to our products. Further, an increasing number of EU countries and other foreign countries use prices for medicinal products established in other countries as "reference prices" to help determine the price of the product in their own territory. Consequently, a downward trend in prices of medicinal products in some countries could contribute to similar downward trends elsewhere. Moreover, in order to obtain reimbursement for our products in some countries, we may be required to conduct clinical trials that compare the cost-effectiveness of our products to other available therapies.

Legally mandated price controls on payment amounts by governmental and private third-party payers or other restrictions could harm our business, results of operations, financial condition and prospects. 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.

For more information regarding government healthcare reform, see "Government Regulation - Health Reform" in Part I, Item 1 of our Annual Report on Form 10-K for the year ended December 31, 2018, filed with the SEC on February 28, 2019.

We face credit risks from government-owned or sponsored 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 healthcare laws or privacy and data protection laws, we may be required to pay penalties, be subjected to scrutiny by regulators or governmental entities, or be suspended from participation in government healthcare programs, which may adversely affect our business, financial condition and results of operations.**

We are subject to various healthcare laws and regulations in the U.S. and internationally, including anti-kickback laws, false claims laws, data privacy and security laws, and laws related to ensuring compliance. In the U.S., 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 healthcare programs, such as Medicare and Medicaid. Under the federal Anti-Kickback Statute and related regulations, certain arrangements are deemed not to violate the federal Anti-Kickback Statute if they fit within a statutory exception or regulatory safe harbor. However, the exceptions 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 exception 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 healthcare services reimbursed by any source, not just governmental payers.

Federal and state false claims laws, including the civil False Claims Act, prohibit any person or entity 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 healthcare benefit program, including private payers, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.

In addition, recent healthcare reform legislation has strengthened these laws in the U.S. 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 civil False Claims Act.

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, integrity, availability, 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. The global data protection landscape is rapidly evolving, and implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future. In the United States, California recently enacted the California Consumer Privacy Act (CCPA), which takes effect on January 1, 2020. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability. Some observers have noted that the CCPA could mark the beginning of a trend toward more stringent privacy legislation in the U.S., which could increase our potential liability and adversely affect our business.

The European Regulation 2016/679, known as the General Data Protection Regulation (GDPR), as well as EU Member State implementing legislations, apply to the collection and processing of personal data, including health-related information, by companies located in the EU, or in certain circumstances, by companies located outside of the EU and processing personal information of individuals located in the EU. These laws impose strict obligations on the ability to process personal data, including health-related information, in particular in relation to their collection, use, disclosure and transfer. These include several requirements relating to (i) obtaining, in some situations, the consent of the individuals to whom the personal data relates, (ii) the information provided to the individuals about how their personal information is used, (iii) ensuring the security and confidentiality of the personal data, (iv) the obligation to notify regulatory authorities and affected individuals of personal data breaches, (v) extensive internal privacy governance obligations, and (vi) obligations to honor rights of individuals in relation to their personal data (for example, the right to access, correct and delete their data). The GDPR prohibits the transfer of personal data to countries outside of the European Economic Area (EEA), such as the United States, which are not considered by the European Commission to provide an adequate level of data protection. Switzerland has adopted similar restrictions. Although there are legal mechanisms to allow for the transfer of personal data from the EEA and Switzerland to the United States, they are subject to legal challenges and uncertainty about compliance with EU data protection laws remains.

Potential pecuniary fines for noncompliant companies may be up to the greater of €20 million or 4% of annual global revenue. The GDPR has increased our responsibility and liability in relation to personal data that we process, and we may be required to put in place additional potential mechanisms to ensure compliance with the new EU data protection rules.

Substantial new provisions affecting compliance have also been adopted in the U.S. and certain foreign countries, which may require us to modify our business practices with healthcare practitioners. For example, in the U.S., the PPACA, through the Physician Payments Sunshine Act, requires certain drug, biologicals and medical supply 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. Effective January 1, 2022, manufacturers will also be required to report on payments or transfers of value to physician assistants, nurse practitioners or clinical nurse specialists, certified registered nurse anesthetists, and certified nurse-midwives. In addition, there has been a recent trend of increased state regulation of payments made to physicians. Certain states and/or local jurisdictions 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, the registration of pharmaceutical sales representatives and/or the tracking and reporting of gifts, compensation and other remuneration to physicians, marketing expenditures, and drug pricing. Likewise, in many foreign countries there is an increasing focus on the relationship between drug companies and healthcare practitioners. Recently enacted legislation creates reporting obligations on payments, gifts and benefits made to these professionals; however, implementing regulations enacting such laws are still pending and subject to varying interpretations by courts and government agencies. The shifting regulatory environment and the need to implement systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the costs of maintaining compliance and the possibility that we may violate one or more of the requirements and be subject to fines or sanctions.

Due to the breadth of the healthcare and privacy and data protection laws described above, the narrowness of available statutory and regulatory exceptions and safe harbors 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. 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, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, curtailment of our operations, and debarment, suspension or exclusion from participation in government healthcare programs, any of which could adversely affect our business, financial condition and results of operations.

***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, Brineura, Kuvan, Naglazyme and Vimizim, and all of the sales of Firdapse are generated from countries other than the U.S. Similarly, we expect a significant portion of the sales of Palynziq to be 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, exposure to fluctuations in foreign currency exchange rates and potential currency controls imposed by foreign governments;
- 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
- rapidly evolving global laws and regulations relating to data protection and the privacy and security of 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.

***The United Kingdom's withdrawal from the European Union may have a negative effect on global economic conditions, financial markets and our business, which could adversely affect our revenue and results of operations.**

In June 2016, a majority of the eligible members of the electorate in the United Kingdom voted to withdraw from the EU in a national referendum (Brexit). On March 29, 2017, the United Kingdom's Prime Minister formally delivered the notice of withdrawal. The withdrawal of the United Kingdom from the EU will take effect on the earlier of the effective date of the withdrawal agreement or January 31, 2020, unless the United Kingdom opts to remain in the EU or the deadline is extended by the EU. It appears likely that this withdrawal will continue to involve lengthy negotiations between the United Kingdom and EU Member States to determine the future terms of the United Kingdom's relationship with the EU and the wider EEA, and it is possible that these negotiations will fail, leading to a "no-deal" Brexit. The U.K. government is currently exploring ways to end the uncertainty over Brexit, including the possibility of a general election in the short-term.

These developments, or the perception that any of them could occur, have had and may continue to have a significant adverse effect on global economic conditions and the stability of global financial markets, and could significantly reduce global market liquidity and restrict the ability of key market participants to operate in certain financial markets. In particular, it could also lead to a period of considerable uncertainty in relation to global financial and banking markets, as well as on regulatory processes in Europe and the EEA. As a result of this uncertainty, global financial markets could experience significant volatility, which could adversely affect the market price of our shares. Asset valuations, currency exchange rates and credit ratings may also be subject to increased market volatility. Lack of clarity about future United Kingdom laws and regulations as the United Kingdom determines which EU rules and regulations to replace or replicate in the event of a withdrawal, including financial laws and regulations, tax and free trade agreements, intellectual property rights, supply chain logistics, environmental, health and safety laws and regulations, immigration laws and employment laws, could decrease foreign direct investment in all markets, increase costs, depress economic activity and restrict access to capital.

If the United Kingdom and the EU are unable to negotiate acceptable withdrawal terms or if other EU countries pursue withdrawal, barrier-free access between the United Kingdom and other EU or EEA countries could be diminished or eliminated, which could make our doing business in the EU more difficult. As a result of Brexit, we may face disruptions in our supply chain, inventory management, manufacturing process and product distribution network, which could adversely affect our business and results of operations. Moreover, Brexit may also lead to new regulatory costs and challenges that could have a material adverse effect on our operations. The EMA has issued guidance to marketing authorization holders of centrally authorized medicinal products regarding certain requirements that need to be considered as part of Brexit, such as the requirement for the marketing authorization holder of a product centrally approved by the EC to be established in the EU, and the requirement for some activities relating to centrally approved products, such as batch release and pharmacovigilance, be performed in the EU. Furthermore, there are few indications of the effect Brexit will have on the pathway to obtaining marketing approval for any of our product candidates in the United Kingdom.

If we fail to comply with U.S. export control and economic sanctions, our business, financial condition and operating results may be adversely affected.

Our products are subject to U.S. export control laws and regulations, including the U.S. Export Administration Regulations and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Control (OFAC). Exports of our products and solutions must be made in compliance with these laws and regulations. Changes to these laws and regulations, or to the countries, governments, persons or activities targeted by such laws, could result in decreased use of our products, or in our decreased ability to export or sell our products to existing or potential customers, which would likely adversely affect our results of operations, financial condition or strategic objectives. If we fail to comply with these laws and regulations, we and certain of our employees could be subject to substantial civil or criminal penalties, including the possible loss of export or import privileges, fines, which may be imposed on us and responsible employees or officers and, in extreme cases, the incarceration of responsible employees or officers.

We rely on a general license from OFAC to sell our medicines for eventual use by hospital and clinic end-users in Iran. The use of this OFAC general license requires us to observe strict conditions with respect to products sold, end-user limitations and payment requirements. Although we believe we have maintained compliance with the general license requirements, there can be no assurance that the general license will not be revoked, be renewed in the future or that we will remain in compliance. A violation of the OFAC general license could result in substantial fines, sanctions, civil or criminal penalties, competitive or reputational harm, litigation or regulatory action and other consequences that might adversely affect our results of operations, financial condition or strategic objectives.

Failure to comply with applicable anti-corruption legislation could result in fines, criminal penalties and materially adversely affect our business, financial condition and results of operations.

We are required to comply with anti-corruption and anti-bribery laws in the jurisdictions in which we operate, including the FCPA in the United States, the U.K. Bribery Act and other similar laws in other countries in which we do business. We operate in a number of countries that are recognized to have a reputation for corruption and pose an increased risk of corrupt practices. We also regularly interact with government regulators in many countries, including those that are considered higher risk for corruption, in order to secure regulatory approval to manufacture and distribute our products. The anti-corruption and anti-bribery laws to which we are subject generally prohibit companies and their intermediaries from making improper payments to foreign officials or other persons for the purposes of influencing official decisions or obtaining or retaining business and/or other benefits. These laws also require us to make and keep books and records that accurately and fairly reflect our transactions and to devise and maintain an adequate system of internal accounting controls. As part of our business, we deal with state-owned business enterprises, the employees and representatives of which may be considered foreign officials for purposes of applicable anti-corruption laws.

Although we have adopted policies and procedures designed to ensure that we, our employees and third-party agents will comply with such laws, there can be no assurance that such policies or procedures will work effectively at all times or protect us against liability under these or other laws for actions taken by our employees, partners and other third parties with respect to our business. If we are not in compliance with anti-corruption laws and other laws governing the conduct of business with government entities and/or officials (including local laws), we may be subject to criminal and civil penalties and other remedial measures, which could harm our business, financial condition, results of operations, cash flows and prospects. Investigations of

any actual or alleged violations of such laws or policies related to us could harm our business, financial condition, results of operations, cash flows and prospects.

Moreover, there has been enhanced scrutiny of company-sponsored patient assistance programs, including insurance premium and co-pay assistance programs and donations to third-party charities that provide such assistance. There has also been enhanced scrutiny by governments on reimbursement support offerings, clinical education programs and promotional speaker programs. If we, our third-party agents or donation recipients are deemed to have failed to comply with laws, regulations or government guidance in any of these areas, we could be subject to criminal or civil sanctions. Any similar violations by our competitors could also negatively impact our industry reputation and increase scrutiny over our business and our products

Changes in funding for the FDA, the EMA and other government agencies or government shutdowns could hinder the ability of such agencies to hire and retain key leadership and other personnel or otherwise prevent those agencies from performing normal functions on which the operation of our business may rely, which could negatively impact our business.

Changes in funding levels of government agencies can affect their ability to hire and retain key personnel and carry out their normal functions that support our business. For example, the ability of the FDA to timely review and approve INDs or marketing authorizations for our product candidates may be hindered by a lack of resources and qualified personnel. In addition, funding of other government agencies on which our operations rely, including those that fund research and development activities, is subject to the political budget process, which is inherently fluid and unpredictable.

Government shutdowns could also impact the ability of government agencies to function normally and support our operations. For example, the U.S. federal government has shut down repeatedly since 1980, including for a period of 35 days beginning on December 22, 2018. During a shutdown, certain regulatory agencies, such as the FDA, have had to furlough key personnel and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

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 United Kingdom Pound, the Canadian Dollar 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 certain 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 Aldurazyme, Naglazyme 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 our products. 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.
- Patents have limited duration and expire. For example, our patents related to Aldurazyme expire in November 2019 and in 2020.
- 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.
- Generic manufacturers may use litigation and regulatory means to obtain approval for generic versions of our products notwithstanding our filed patents or patent applications.
- Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing products, which could increase our operating expenses and delay product programs.
- 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.

Under policies recently adopted in the EU, clinical trial data submitted to the EMA in MAAs that were traditionally regarded as confidential commercial information are now subject to public disclosure. Subject to our ability to review and redact a narrow sub-set of confidential commercial information, the new EU policies will result in the EMA's public disclosure of certain of our clinical study reports, clinical trial data summaries and clinical overviews for recently completed and future MAA submissions. 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 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 valoctocogene roxaparvovec, 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 depend 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.

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 Aldurazyme, Firdapse, Kuvan and Naglazyme. 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 are successful in their use of litigation or regulatory means to obtain approval for generic versions of Kuvan, our revenue and results of operations would be adversely affected.

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

Pursuant to the Hatch-Waxman Act, companies were permitted to file ANDA applications for proposed generic versions 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.

We received separate paragraph IV notice letters in 2016, 2015 and 2014 from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL) and Par Pharmaceutical, Inc. (Par) notifying us that each of DRL and Par had filed ANDAs seeking approval of proposed generic versions of Kuvan (sapropterin dihydrochloride) 100 mg oral powder and Kuvan 100 mg oral tablets prior to the expiration of our Kuvan-related patents listed in the Orange Book. We filed lawsuits alleging patent infringement against DRL and Par, and in 2017, 2016 and 2015 we entered into separate settlement agreements with DRL (the DRL Settlement Agreement) and Par (the Par Settlement Agreement) that resolved the patent litigation in the U.S. Under the terms of the DRL Settlement Agreement, we granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride in 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances. Under the Par Settlement Agreement, we granted Par a non-exclusive license to our Kuvan-related patents to allow Par to market a generic version of sapropterin dihydrochloride in 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on: April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances.

We expect generic versions of Kuvan to first become available in the U.S. in the fourth quarter of 2020. The DRL Settlement Agreement and the Par Settlement Agreement, as well as any future ANDA or related legal proceeding, 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 any ANDA filer we bring suit against 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 and Par following the settlements 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, and there is a process equivalent to the ANDA process under Article 10 of Directive 2001/83/EC in the EU. 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 license or acquire in the future may be intended for patient populations that are significantly larger than any of the patient populations we currently target. 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 product candidates are approved, if doctors elect a course of treatment which does not include our products, this decision would reduce demand for our 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 Aldurazyme, Naglazyme, and Vimizim in MPS diseases, could be greatly reduced. Moreover, if we obtain regulatory approval for valoctocogene roxaparvovec, the commercial success of valoctocogene roxaparvovec will still depend, in part, on the acceptance of physicians, patients and healthcare payers 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 operations, 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 or attacks, could harm our ability to operate our business effectively. Our ability to manage and maintain our operations, 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 incidents and attacks in particular are evolving and include, but are not limited to, malicious software, attempts to gain unauthorized access to data, business email compromise and other cyber attacks or cyber incidents that could lead to disruptions in or unavailability of systems, misappropriation of confidential or otherwise protected information, corruption or loss of data, data security breaches and other harm to our business or competitive position. 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 operations, inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. Moreover, if such an incident or computer security breach were to result in damage or unauthorized access to, or loss, corruption or unauthorized disclosure of, personally identifiable information, such a breach may require notification to governmental agencies, supervisory bodies, credit reporting agencies, the media or individuals pursuant to various federal, state and foreign data protection, privacy and security laws, regulations and guidelines, if applicable. It could also cause a loss in the confidence of our customers, employees, and partners and other third parties with respect to our business. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary, personal and confidential information, including research or clinical data and information about patients, employees, contractors and others, could require significant capital investments to remediate and could adversely affect our business, financial condition and results of operations. We would also be exposed to a risk of loss, enforcement measures, penalties, fines, indemnification claims or litigation and potential civil or criminal liability, which could materially adversely affect our business, financial condition and results of operations.

If a natural disaster or terrorist or criminal activity caused significant damage to our facilities or the facilities of our third-party manufacturers and suppliers, 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 currently manufacture Aldurazyme, Brineura, Naglazyme, Palynziq 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 Aldurazyme, Brineura, Naglazyme and Vimizim or our third-party manufacturers' ability to manufacture Firdapse and Kuvan.

Our Galli Drive facility, located in Novato, California, is currently our only manufacturing facility for Aldurazyme, Naglazyme and Palynziq and is one of two manufacturing facilities for Brineura and Vimizim. Our gene therapy manufacturing facility is also located in Novato, California, and it is currently our only manufacturing facility to support valoctocogene roxaparvovec clinical development activities and the anticipated commercial demand for valoctocogene roxaparvovec, if approved. These facilities are located in the San Francisco Bay Area near known earthquake fault zones and are 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, explosions, 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 our products, or to have our products 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.

The impact of the recently passed U.S. comprehensive tax reform bill on us is uncertain and could have a material adverse effect on our business and financial condition.

On December 22, 2017, the U.S. President signed into law new legislation, known as the Tax Cuts & Jobs Act, which significantly revises the Internal Revenue Code of 1986, as amended. The newly enacted federal income tax law, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, creation of a base erosion and anti-abuse tax and modification or repeal of many business deductions and credits (including reduction of tax credits under the Orphan Drug Act). Many aspects of the new federal tax law are unclear and may not be clarified for some time. Notwithstanding the reduction in the corporate income tax rate, it is possible that the Tax Cuts & Jobs Act, or regulations or interpretations under it, could adversely affect our business and financial condition, and such effect could be material. In addition, it is uncertain if and to what extent various U.S. states will conform to the newly enacted federal tax law.

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 healthcare 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.

We sell our products in countries that face economic volatility and weakness. Although we have 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 our products. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected.

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

Risks Related to Ownership of Our Securities

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

Our valuation and stock price 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 our products;
- 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;
- generic competition to Kuvan tablets and powder relating to our settlements with DRL and Par or potential generic competition from future competitors;
- government regulatory action affecting our product candidates, our products or our competitors' product candidates and 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;
- negative publicity about us or the pharmaceutical industry;
- changes in the structure of healthcare payment systems;
- cybersecurity incidents experienced by us or others in our industry;
- 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 orders for our products, in particular in Latin America, where governments place large periodic orders for Naglazyme and Vimizim;
- 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 stockholders, 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 in the future become 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 2020 Notes, we entered into capped call transactions with respect to 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 2020 Notes and are expected generally to reduce potential dilution to the common stock upon conversion of the 2020 Notes in excess of the principal amount of such converted 2020 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 2020 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 2020 Notes (and are likely to do so during the settlement averaging period under the capped call transactions, which precedes the maturity date of the 2020 Notes, and on or around any earlier conversion date related to a conversion of the 2020 Notes).

The effect, if any, of any of these transactions and activities on the market price of our common stock or the 2020 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 2020 Notes and the value of our common stock, if any, that 2020 Note holders receive upon any conversion of the 2020 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 us more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our restated certificate of incorporation and amended and restated bylaws providing that stockholders' meetings may only be called by our Chairman, the lead independent director or the majority of our Board of Directors and 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 us. 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 us over.

The terms of the Notes require us to repurchase the Notes in the event of a fundamental change. A takeover of us 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 us that would otherwise be beneficial to our stockholders or investors in the Notes.

***Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware will be the exclusive forum for the adjudication of certain disputes, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.**

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee of BioMarin to us or our stockholders;
- any action asserting a claim against us or any of our directors, officers or other employees arising pursuant to any provision of the General Corporation Law of the State of Delaware, our restated certificate of incorporation or our amended and restated bylaws; and
- any action asserting a claim against us or any of our directors, officers or other employees that is governed by the internal affairs doctrine.

This exclusive-forum provision would not apply to suits brought to enforce a duty or liability created by the Securities Act of 1933, as amended, the Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction, and further provides that any person or entity that acquires any interest in shares of our capital stock will be deemed to have notice of

and consented to the provisions of such provision, including consent to the personal jurisdiction of the Court of Chancery of the State of Delaware related to any action covered by such provision.

This exclusive-forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers, and other employees. If a court were to find this exclusive-forum provision to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information.

None.

| Item 6. | Exhibits. |
|------------------------|---|
| <u>Exhibit Number</u> | <u>Description</u> |
| 2.1 | 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 January 7, 2016 as Exhibit 2.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 2.2 | Termination Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 2.3 | 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 January 7, 2016 as Exhibit 2.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 2.4 | First Amendment, dated as of December 12, 2016, to the Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015 and effective as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on February 27, 2017 as Exhibit 2.6 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 3.1 | Restated Certificate of Incorporation of BioMarin Pharmaceutical Inc., previously filed with the SEC on June 12, 2017 as Exhibit 3.2 to the Company's Current Report on Form 8-K (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 September 24, 2018 as Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 10.1* | BioMarin Pharmaceutical Inc. Summary of Independent Director Compensation |
| 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 - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document. |
| 101.SCH | Inline XBRL Taxonomy Extension Schema Document |
| 101.CAL | Inline XBRL Taxonomy Extension Calculation Document |
| 101.DEF | Inline XBRL Taxonomy Extension Definition Linkbase |
| 101.LAB | Inline XBRL Taxonomy Extension Labels Linkbase Document |

101.PRE Inline XBRL Taxonomy Extension Presentation Link Document

104 XBRL tags for the cover page from the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, are embedded within the Inline XBRL document.

* Filed herewith

+ The certifications attached as Exhibit 32.1 accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed "filed" by the Registrant for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any of the Registrant's filings under the Securities Act of 1933, as amended, irrespective of any general incorporation language contained in any such filing.

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, 2019 and December 31, 2018, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three and nine months ended September 30, 2019 and 2018, (iii) Condensed Consolidated Statement of Stockholders' Equity for the three and nine months ended September 30, 2019 and 2018, (iv) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2019 and 2018, and (v) Notes to Condensed Consolidated Financial Statements.

BioMarin Pharmaceutical Inc.
Summary of Independent Director Compensation

Effective as of September 19, 2019, the Board of Directors (the “Board”) of BioMarin Pharmaceutical Inc. (the “Company”) approved revised compensation for the independent directors of the Company. All independent directors are entitled to receive a combination of annual cash retainers and restricted stock unit (“RSU”) grants as summarized below as compensation for their service on the Company’s Board and Board committees.

Cash Compensation

The following table is a summary of the annual cash compensation payable to independent directors. Each applicable line item is an additional element of compensation.

| Director Position | Annual Cash Compensation |
|--|---------------------------------|
| All Independent Directors | \$ 65,000 |
| <i>Additional Elements of Compensation:</i> | |
| Independent Chair of the Board (if applicable) | \$ 65,000 |
| Lead Independent Director (if applicable) | \$ 65,000 |
| Audit Committee Member | \$ 13,500 |
| Audit Committee Chair (premium in addition to committee membership retainer) | \$ 13,000 |
| Compensation Committee Member | \$ 10,000 |
| Compensation Committee Chair (premium in addition to committee membership retainer) | \$ 10,000 |
| Corporate Governance and Nominating Committee Member | \$ 8,750 |
| Corporate Governance and Nominating Committee Chair (premium in addition to committee membership retainer) | \$ 10,000 |
| Science and Technology Committee Member | \$ 10,000 |
| Science and Technology Committee Chair (premium in addition to committee membership retainer) | \$ 10,000 |

Equity Compensation

Annual Award

On the date of our annual meeting of stockholders for a given year, each reelected director receives an RSU grant valued at \$400,000, based on the Black-Scholes model valuation using a three-month trailing average closing price of our common stock. The shares of common stock subject to the Annual Award vest in full on the one-year anniversary of the grant date, subject to each respective director providing service to the Company through such vesting date.

New Independent Directors

Upon election or appointment, a new independent director will receive an RSU grant on the same terms as the Annual Award, pro-rated for amount and vesting to the nearest quarter for the time such new director will serve prior to the Company’s next Annual Meeting of Stockholders.

CERTIFICATION

I, Jean-Jacques Bienaimé, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of BioMarin Pharmaceutical Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

October 29, 2019

/s/ JEAN-JACQUES BIENAIMÉ

Jean-Jacques Bienaimé
Chief Executive Officer

CERTIFICATION

I, Daniel Spiegelman, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of BioMarin Pharmaceutical Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

October 29, 2019

/s/ DANIEL SPIEGELMAN

Daniel Spiegelman
Executive Vice President and Chief Financial Officer

**CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

We, Jean-Jacques Bienaimé and Daniel Spiegelman, hereby certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that BioMarin Pharmaceutical Inc.'s Quarterly Report on Form 10-Q for the period ended September 30, 2019, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and the information contained in such Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of BioMarin Pharmaceutical Inc.

/s/ JEAN-JACQUES BIENAIMÉ

Jean-Jacques Bienaimé
Chief Executive Officer

October 29, 2019

/s/ DANIEL SPIEGELMAN

Daniel Spiegelman
Executive Vice President and Chief Financial Officer

October 29, 2019

This certification accompanies the Quarterly Report on Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of BioMarin Pharmaceutical Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Quarterly Report on Form 10-Q), irrespective of any general incorporation language contained in such filing.