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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

Form 10-Q

(Mark One)

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

For the quarterly period ended June 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	**68-0397820**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

770 Lindaro Street	**San Rafael**	**California**	**94901**
(Address of principal executive offices)			**(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	☒	Accelerated Filer	☐
Non-accelerated Filer	☐	Smaller Reporting Company	☐
Emerging Growth Company	☐		

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: 179,462,759 shares of common stock, par value $0.001, outstanding as of July 22, 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.

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
June 30, 2019 and December 31, 2018
(In thousands, except share and per share amounts)

	June 30, 2019	December 31, 2018(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 307,577	$ 493,982
Short-term investments	423,526	590,326
Accounts receivable, net	377,150	342,633
Inventory	578,736	530,871
Other current assets	119,779	98,403
Total current assets	1,806,768	2,056,215
Noncurrent assets:		
Long-term investments	374,965	235,864
Property, plant and equipment, net	962,970	948,682
Intangible assets, net	476,632	491,808
Goodwill	197,039	197,039
Deferred tax assets	475,554	460,952
Other assets	99,456	36,568
Total assets	$ 4,393,384	$ 4,427,128
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 429,502	$ 437,290
Short-term contingent consideration	9,926	85,951
Total current liabilities	439,428	523,241
Noncurrent liabilities:		
Long-term convertible debt, net	839,165	830,417
Long-term contingent consideration	50,151	46,883
Other long-term liabilities	103,686	58,647
Total liabilities	1,432,430	1,459,188
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 179,433,316 and 178,252,954 shares issued and outstanding, respectively.	179	178
Additional paid-in capital	4,744,316	4,669,926
Company common stock held by Nonqualified Deferred Compensation Plan (the NQDC)	(10,211)	(13,301)
Accumulated other comprehensive income	17,439	5,271
Accumulated deficit	(1,790,769)	(1,694,134)
Total stockholders' equity	2,960,954	2,967,940
Total liabilities and stockholders' equity	$ 4,393,384	$ 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 LOSS
Three and Six Months Ended June 30, 2019 and 2018
(In thousands, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
REVENUES:				
Net product revenues	$ 379,075	$ 367,786	$ 773,558	$ 736,885
Royalty and other revenues	8,688	5,059	14,950	9,407
Total revenues	387,763	372,845	788,508	746,292
OPERATING EXPENSES:				
Cost of sales	77,436	79,019	166,618	161,352
Research and development	185,641	175,582	369,232	359,530
Selling, general and administrative	160,754	153,280	322,912	291,616
Intangible asset amortization and contingent consideration	20,286	10,227	40,051	23,429
Gain on sale of intangible assets	(15,000)	(20,000)	(15,000)	(20,000)
Total operating expenses	429,117	398,108	883,813	815,927
LOSS FROM OPERATIONS	(41,354)	(25,263)	(95,305)	(69,635)
Equity in the loss of BioMarin/Genzyme LLC	(44)	(107)	(229)	(39)
Interest income	5,899	5,569	12,197	10,803
Interest expense	(6,866)	(12,225)	(13,593)	(23,787)
Other income, net	470	2,849	2,078	2,677
LOSS BEFORE INCOME TAXES	(41,895)	(29,177)	(94,852)	(79,981)
Benefit from income taxes	(4,460)	(12,385)	(944)	(19,040)
NET LOSS	$ (37,435)	$ (16,792)	$ (93,908)	$ (60,941)
NET LOSS PER SHARE, BASIC AND DILUTED	$ (0.21)	$ (0.09)	$ (0.53)	$ (0.35)
Weighted average common shares outstanding, basic and diluted	179,048	176,873	178,662	176,405
COMPREHENSIVE (LOSS)/INCOME	$ (39,790)	$ 10,624	$ (81,740)	$ (38,523)

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

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENT OF STOCKHOLDERS' EQUITY
Three and Six Months Ended June 30, 2019 and 2018
(In thousands)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Shares of Common Stock				
Beginning balance at March 31, 2019 and 2018 and December 31, 2018 and December 31, 2017, respectively [1]	179,033	176,653	178,253	175,844
Issuances under equity incentive plans	400	855	1,180	1,664
Ending balance	179,433	177,508	179,433	177,508
Beginning balance at March 31, 2019 and 2018 and December 31, 2018 and December 31, 2017, respectively [1]	$ 2,967,940	$ 2,808,663	$ 2,967,940	$ 2,808,663
Common stock:				
Beginning balance [1]	179	177	178	176
Issuances under equity incentive plans, net of tax	—	1	1	2
Ending balance	179	178	179	178
Additional paid-in capital:				
Beginning balance [1]	4,682,900	4,510,451	4,669,926	4,483,220
Issuances under equity incentive plans, net of tax	10,843	26,076	(17,889)	16,310
Stock-based compensation	40,362	40,773	82,068	77,770
Common stock held by the NQDC	(442)	—	(442)	
Accounting impact of NQDC Plan change (See Note 9)	10,653	—	10,653	—
Ending balance	4,744,316	4,577,300	4,744,316	4,577,300
Company common stock held by the NQDC:				
Beginning balance [1]	(12,912)	(14,017)	(13,301)	(14,224)
Common stock held by the NQDC	53	627	442	834
Accounting impact of NQDC Plan change (See Note 9)	2,648	—	2,648	—
Ending balance	(10,211)	(13,390)	(10,211)	(13,390)
Accumulated other comprehensive income (loss):				
Beginning balance [1]	19,794	(28,545)	5,271	(22,961)
Impact of changes in accounting principle	—	—	—	(586)
Other comprehensive income (loss)	(2,355)	27,416	12,168	22,418
Ending balance	17,439	(1,129)	17,439	(1,129)
Accumulated Deficit:				
Beginning balance [1]	(1,753,334)	(1,661,063)	(1,694,134)	(1,637,548)
Impact of changes in accounting principles	—	—	(2,727)	20,634
Net loss	(37,435)	(16,792)	(93,908)	(60,941)
Ending balance	(1,790,769)	(1,677,855)	(1,790,769)	(1,677,855)
Total stockholders' equity, ending balances at June 30, 2019 and June 30, 2018, respectively	$ 2,960,954	$ 2,885,104	$ 2,960,954	$ 2,885,104

(1) The beginning balances for the six 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
Six Months Ended June 30, 2019 and 2018
(In thousands)
(unaudited)

	Six Months Ended June 30,	
	2019	**2018**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (93,908)	$ (60,941)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	48,771	44,610
Non-cash interest expense	8,870	17,300
Accretion of discount on investments	(1,510)	(41)
Stock-based compensation	82,590	75,214
Gain on sale of intangible assets	(15,000)	(20,000)
Deferred income taxes	(10,083)	(29,681)
Unrealized foreign exchange (gain) loss	799	(5,693)
Non-cash changes in the fair value of contingent consideration	4,891	1,828
Other	(3,603)	1,772
Changes in operating assets and liabilities:		
Accounts receivable, net	(31,030)	(77,416)
Inventory	(33,119)	15,493
Other current assets	(4,989)	(2,037)
Other assets	(4,593)	(6,448)
Accounts payable and accrued liabilities	(37,571)	(32,989)
Other long-term liabilities	10,279	2,663
Net cash used in operating activities	(79,206)	(76,366)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(61,387)	(52,682)
Maturities and sales of investments	423,477	311,969
Purchases of available-for-sale securities	(386,320)	(345,458)
Proceeds from sale of intangible asset	—	20,000
Purchase of intangible assets	(5,770)	—
Other	(808)	(841)
Net cash used in investing activities	(30,808)	(67,012)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of awards under equity incentive plans	19,013	44,926
Taxes paid related to net share settlement of equity awards	(36,900)	(28,614)
Payment of contingent acquisition consideration	(57,507)	(43,108)
Other	(1,347)	—
Net cash used in financing activities	(76,741)	(26,796)
Effect of exchange rate changes on cash	350	(443)
NET DECREASE IN CASH AND CASH EQUIVALENTS	(186,405)	(170,617)
Cash and cash equivalents:		
Beginning of period	$ 493,982	$ 598,028
End of period	$ 307,577	$ 427,411
SUPPLEMENTAL CASH FLOW DISCLOSURES:		
Cash paid for income taxes	$ 3,933	$ 14,858
Cash paid for interest	4,295	5,831
SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON CASH INVESTING AND FINANCING ACTIVITIES:		
Decrease in accounts payable and accrued liabilities related to fixed assets	$ (3,467)	$ (7,734)

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 six months ended June 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.

(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 three and six months ended June 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 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 six months ended June 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.

(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 June 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 June 30, 2019 and December 31, 2018, respectively:

	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	$ 226,959	$ —	$ —	$ 226,959	$ 226,959	$ —	$ —
Level 2:							
Money market instruments	68,648	—	—	68,648	68,648	—	—
Corporate debt securities	564,624	3,270	(216)	567,678	—	279,033	288,645
Commercial paper	16,972	—	—	16,972	8,981	7,991	—
U.S. government agency securities	224,071	1,185	(141)	225,115	2,989	136,502	85,624
Foreign and other	550	147	(1)	696	—	—	696
Subtotal	874,865	4,602	(358)	879,109	80,618	423,526	374,965
Total	$ 1,101,824	$ 4,602	$ (358)	$ 1,106,068	$ 307,577	$ 423,526	$ 374,965

	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	$ 1,322,661	$ 497	$ (2,986)	$ 1,320,172	$ 493,982	$ 590,326	$ 235,864

(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 June 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.

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

(6) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	June 30, 2019	December 31, 2018
Intangible assets:		
Finite-lived intangible assets	$ 640,125	$ 307,995
Indefinite-lived intangible assets	—	326,359
Gross intangible assets:	640,125	634,354
Less: Accumulated amortization	(163,493)	(142,546)
Net carrying value	$ 476,632	$ 491,808

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 was put in use with an estimated useful life of approximately nine years, resulting from the completion of Palynziq program development upon receiving European regulatory approval.

During the second quarter of 2019, the Company recorded $15.0 million of gain on sale of intangible assets in the Consolidated Statements of Comprehensive 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:

	June 30, 2019	December 31, 2018
Building and improvements	$ 701,060	$ 694,447
Manufacturing and laboratory equipment	357,049	345,947
Computer hardware and software	162,696	157,787
Leasehold improvements	50,502	41,188
Furniture and equipment	36,229	33,234
Land improvements	7,253	6,551
Land	77,994	77,993
Construction-in-progress	85,189	64,170
	1,477,972	1,421,317
Accumulated depreciation	(515,002)	(472,635)
Total property, plant and equipment, net	$ 962,970	$ 948,682

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 six months ended June 30, 2019 was $21.5 million and $43.0 million, respectively, of which $8.6 million and $15.1 million, respectively, was capitalized into inventory. Depreciation expense for the three and six months ended June 30, 2018 was $20.1 million and $40.1 million, respectively, of which $6.6 million and $10.5 million, respectively, was capitalized into inventory.

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

(8) SUPPLEMENTAL BALANCE SHEET INFORMATION

Inventory consisted of the following:

	June 30, 2019	December 31, 2018
Raw materials	$ 67,397	$ 74,616
Work-in-process	303,231	231,064
Finished goods	208,108	225,191
Total inventory	$ 578,736	$ 530,871

Inventory as of June 30, 2019, included manufacturing-related costs for the commercial production of valoctocogene roxaparvovec inventory totaling $1.9 million. Valoctocogene roxaparvovec is an investigational gene therapy product candidate to treat severe hemophilia A. The Company must receive marketing approval from the applicable regulators before the valoctocogene roxaparvovec inventory can be sold commercially. The Company believes that all material uncertainties related to the ultimate regulatory approval of valoctocogene roxaparvovec 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 roxaparvovec will be expensed.

Accounts Payable and Accrued Liabilities consisted of the following:

	June 30, 2019	December 31, 2018
Accounts payable and accrued operating expenses	$ 223,947	$ 207,620
Accrued compensation expense	101,111	149,937
Accrued rebates payable	49,183	43,116
Accrued royalties payable	23,059	19,977
Value added taxes payable	9,196	7,785
Forward foreign currency exchange contracts	6,423	4,178
Lease liability	10,165	—
Other	6,418	4,677
Total accounts payable and accrued liabilities	$ 429,502	$ 437,290

(9) FAIR VALUE MEASUREMENTS

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

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

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. There were no financial assets or liabilities that were remeasured using a quoted price in active markets for identical assets (Level 1) as of June 30, 2019.

	Fair Value Measurements at June 30, 2019		
	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:			
Other current assets:			
NQDC Plan assets	$ 1,116	$ —	$ 1,116
Other assets:			
NQDC Plan assets	15,003	—	15,003
Restricted investments [1]	2,713	—	2,713
Total other assets	17,716	—	17,716
Total assets	$ 18,832	$ —	$ 18,832
Liabilities:			
Current liabilities:			
NQDC Plan liability [2]	$ 1,116	$ —	$ 1,116
Contingent consideration	—	9,926	9,926
Total current liabilities	1,116	9,926	11,042
Other long-term liabilities:			
NQDC Plan liability [2]	15,003	—	15,003
Contingent consideration	—	50,151	50,151
Total other long-term liabilities	15,003	50,151	65,154
Total liabilities	$ 16,119	$ 60,077	$ 76,196

(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 June 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, all shares were sold and an immaterial gain was realized.

There were no transfers between levels during the three and six months ended June 30, 2019. Liabilities measured at fair value on a recurring basis using Level 3 inputs includes contingent consideration.

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		19,161
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		(531)
Contingent consideration at June 30, 2019	$	60,077

(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 June 30, 2019 (notional amounts in millions):

Foreign Exchange Contracts	Number of Contracts	Aggregate Notional Amount in Foreign Currency	Maturity
Australian Dollars – Sell	12	5.6	July 2019 - Dec. 2019
Brazilian Reais – Sell	3	40.4	Aug 2019
Canadian Dollars – Sell	12	17.9	July 2019 - Dec. 2019
Colombian Pesos – Sell	12	98,000.0	July 2019 - June 2020
Euros – Purchase	154	194.0	July 2019 - June 2022
Euros – Sell	466	572.6	July 2019 - June 2022
Norwegian Krone – Sell	6	23.4	July 2019 - Dec. 2019
Total	665		

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

Foreign Exchange Contracts	Number of Contracts	Aggregate Notional Amount in Foreign Currency	Maturity
Colombian Pesos – Sell	2	69,000.0	July 2019 - Aug. 2019
Euros – Purchase	2	35.7	July 2019 - Aug. 2019
Euros - Sell	2	5.4	July 2019
Great British Pounds - Purchase	2	18.2	July 2019 - Aug. 2019
Great British Pounds - Sell	1	7.6	July 2019
Rubles – Sell	2	2,010.0	July 2019 - Aug. 2019
Total	11		

(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	June 30, 2019	December 31, 2018
Derivatives designated as hedging instruments:		
Asset Derivatives - Level 2 (1)		
Other current assets	$ 15,577	$ 12,686
Other assets	13,214	10,324
Subtotal	$ 28,791	$ 23,010
Liability Derivatives - Level 2 (1)		
Accounts payable and accrued liabilities	$ 5,250	$ 4,036
Other long-term liabilities	4,988	3,653
Subtotal	$ 10,238	$ 7,689
Derivatives not designated as hedging instruments:		
Asset Derivatives - Level 2 (1)		
Other current assets	$ 614	$ 168
Other assets	—	—
Subtotal	$ 614	$ 168
Liability Derivatives - Level 2 (1)		
Accounts payable and accrued liabilities	$ 1,173	$ 142
Other long-term liabilities	—	—
Subtotal	$ 1,173	$ 142
Total Derivatives Asset	$ 29,405	$ 23,178
Total Derivatives Liabilities	$ 11,411	$ 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 June 30, 2019	Six Months Ended June 30, 2019
Amount of Gain (Loss) Recognized in Other Comprehensive Income	$ (1,367)	$ 11,458

	Three Months Ended June 30, 2019		Six Months Ended June 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	$ 379,075	$ 4,280	$ 773,558	$ 4,975
Operating expenses as reported	$ 429,117	$ (768)	$ 883,813	$ (497)
Derivatives Not Designated as Hedging Instruments		Gains (Losses) Recognized in Earnings		Gains (Losses) Recognized in Earnings
Operating Expenses		$ (918)		$ (3,896)

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

As of June 30, 2019, the Company expects to reclassify unrealized gains of $7.6 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 all of 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 June 30, 2019:

Lease Classification	Classification	June 30, 2019
Assets:		
Operating	Other Assets	$ 51,196
Financing	Other Assets	8,599
Total ROU assets		$ 59,795
Liabilities:		
Current:		
Operating	Accounts payable and accrued liabilities	$ 7,277
Financing	Accounts payable and accrued liabilities	2,888
Noncurrent:		
Operating	Other long-term liabilities	45,921
Financing	Other long-term liabilities	7,958
Total lease liabilities		$ 64,044

Maturities of lease liabilities as of June 30, 2019 by fiscal year are as follows:

Maturity of Lease Liabilities	Operating	Financing	Total
2019	$ 5,418	$ 1,683	$ 7,101
2020	9,518	3,466	12,984
2021	8,533	2,866	11,399
2022	8,205	2,259	10,464
2023	6,773	1,747	8,520
Thereafter	27,942	—	27,942
Total lease payments	66,389	12,021	78,410
Less: Interest	(13,191)	(1,175)	(14,366)
Present value of lease liabilities	$ 53,198	$ 10,846	$ 64,044

Lease Cost	Classification	Three Months Ended June 30, 2019	Six Months Ended June 30, 2019
Operating [1]	Operating Expenses	$ 3,233	$ 6,418
Financing:			
Amortization	Operating Expenses	605	1,211
Interest expense	Operating Expenses	152	313
Total lease costs		$ 3,990	$ 7,942

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

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

Other Information	June 30, 2019
Weighted average remaining lease term (in years):	
Operating leases	8.2
Financing leases	3.8
Weighted average discount rate:	
Operating leases	5.2%
Financing leases	5.4%
Additional leases not yet commenced (undiscounted):	
Operating lease liability to commence in the second half of 2019	$ 973

Supplemental Cash Flow Information	Six Months Ended June 30, 2019
Cash paid for amounts included in the measurement of lease liabilities:	
Cash used in operating activities:	
Operating leases	$ 3,712
Financing leases	$ 312
Cash used in financing activities:	
Financing leases	$ 1,346
ROU assets obtained in exchange for lease obligations:	
Operating leases	$ 8,909
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.

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

(12) DEBT

Convertible Notes

As of June 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, quarterly. The following table summarizes information regarding the Company's convertible debt:

	June 30, 2019	December 31, 2018
1.50% senior subordinated convertible notes due in October 2020 (the 2020 Notes)	374,993	374,993
Unamortized discount	(19,432)	(26,581)
Unamortized deferred offering costs	(1,684)	(2,334)
Convertible Notes due in 2020, net	353,877	346,078
0.599% senior subordinated convertible notes due in August 2024 (the 2024 Notes)	495,000	495,000
Unamortized discount	(7,240)	(7,946)
Unamortized deferred offering costs	(2,472)	(2,715)
Convertible Notes due in 2024, net	485,288	484,339
Total convertible debt, net	$ 839,165	$ 830,417
Fair value of fixed rate convertible debt		
Convertible Notes due in October 2020 [(1)]	420,727	419,722
Convertible Notes due in August 2024 [(1)]	512,582	491,626
Total fair value of fixed rate convertible debt	$ 933,309	$ 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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Coupon interest expense	$ 2,254	$ 3,527	$ 4,411	$ 6,488
Amortization of debt issuance costs	508	1,006	1,015	2,010
Accretion of discount on convertible notes	3,953	7,692	7,855	15,289
Total interest expense on convertible debt	$ 6,715	$ 12,225	$ 13,281	$ 23,787

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.

(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) and terminated the 2016 Credit Facility, which had availability of up to $100.0 million in revolving loans. 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 June 30, 2019 and December 31, 2018, there were no outstanding amounts due on nor any usage of the 2018 Credit Facility. As of June 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 Loss for the three and six months ended June 30, 2019 and 2018.

	Three Months Ended June 30,		Six Months Ended June 30,		Condensed Consolidated Statement of Comprehensive Loss Classification
	2019	2018	2019	2018	
Gains (losses) on cash flow hedges:					
Forward contracts	$ 4,280	$ (4,062)	$ 4,975	$ (11,708)	Net product revenues
Forward contracts	(768)	1,403	(497)	3,264	Operating expenses
Total gain (loss) on cash flow hedges	$ 3,512	$ (2,659)	$ 4,478	$ (8,444)	

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 six months ended June 30, 2019 and 2018.

	Three Months Ended June 30, 2019			
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Other	Total
AOCI balance at March 31, 2019	$ 19,060	$ 748	$ (14)	$ 19,794
Other comprehensive income before reclassifications	(1,367)	3,279	1	1,913
Less: net gain (loss) reclassified from AOCI	3,512	—	—	3,512
Tax effect	—	(756)	—	(756)
Net current-period other comprehensive income (loss)	(4,879)	2,523	1	(2,355)
AOCI balance at June 30, 2019	$ 14,181	$ 3,271	$ (13)	$ 17,439

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

	Six Months Ended June 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 before reclassifications	11,458	6,734	—	18,192
Less: gain (loss) reclassified from AOCI	4,478	—	—	4,478
Tax effect	—	(1,546)	—	(1,546)
Net current-period other comprehensive income (loss)	6,980	5,188	—	12,168
AOCI balance at June 30, 2019	$ 14,181	$ 3,271	$ (13)	$ 17,439

	Three Months Ended June 30, 2018			
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available for-Sale Debt Securities	Other	Total
AOCI balance at March 31, 2018	$ (23,673)	$ (4,866)	$ (6)	$ (28,545)
Other comprehensive income (loss) before reclassifications	23,582	1,531	(5)	25,108
Less: gain (loss) reclassified from AOCI	(2,659)	—	—	(2,659)
Tax effect	—	(351)	—	(351)
Net current-period other comprehensive income (loss)	26,241	1,180	(5)	27,416
AOCI balance at June 30, 2018	$ 2,568	$ (3,686)	$ (11)	$ (1,129)

	Six Months Ended June 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	14,356	(490)	(4)	13,862
Less: gain (loss) reclassified from AOCI	(8,444)	—	—	(8,444)
Tax effect	—	112	—	112
Net current-period other comprehensive income (loss)	22,800	(378)	(4)	22,418
AOCI balance at June 30, 2018	$ 2,568	$ (3,686)	$ (11)	$ (1,129)

(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

(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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Total revenues by geographic region:				
United States	$ 169,407	$ 162,789	$ 360,343	$ 353,360
Europe	119,561	107,221	244,100	212,871
Latin America	46,494	38,152	80,333	76,555
Rest of world	52,301	64,683	103,732	103,506
Total revenues	$ 387,763	$ 372,845	$ 788,508	$ 746,292

The following table disaggregates Net Product Revenues by product.

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Net product revenues by product:				
Aldurazyme	$ 5,826	$ 24,003	$ 51,093	$ 90,059
Brineura	14,795	10,890	26,975	17,807
Firdapse	5,482	5,177	10,594	10,103
Kuvan	113,323	109,045	220,247	208,160
Naglazyme	98,127	91,086	185,054	166,082
Palynziq	18,836	—	31,108	—
Vimizim	122,686	127,585	248,487	244,674
Total net product revenues	$ 379,075	$ 367,786	$ 773,558	$ 736,885

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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
United States	$ 160,918	$ 138,411	$ 305,203	$ 262,552
Europe	113,593	102,621	236,678	207,419
Latin America	46,493	48,844	80,333	76,555
Rest of world	52,245	53,907	100,251	100,300
Total net product revenues marketed by the Company	373,249	343,783	722,465	646,826
Aldurazyme net product revenues marketed by Genzyme	5,826	24,003	51,093	90,059
Total net product revenues	$ 379,075	$ 367,786	$ 773,558	$ 736,885

(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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Customer A	16%	18%	17%	18%
Customer B	13%	12%	12%	12%
Customer C	12%	10%	11%	9%
Customer D	2%	7%	7%	12%
Total	43%	47%	47%	51%

On a consolidated basis, two customers accounted for 25% and 12% of the June 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 June 30, 2019 and December 31, 2018, the accounts receivable balance for Genzyme included $69.9 million and $73.9 million, respectively, of unbilled accounts receivable, which become payable to the Company when the product is sold through 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 Loss for all stock-based compensation arrangements was as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Cost of sales	$ 3,717	$ 3,246	$ 8,536	$ 6,386
R&D	14,943	15,573	28,776	28,842
Selling, general and administrative	21,169	19,787	45,278	39,986
Total stock-based compensation expense	$ 39,829	$ 38,606	$ 82,590	$ 75,214

Stock-based compensation of $4.3 million and $8.1 million was capitalized into inventory for the three and six months ended June 30, 2019, respectively, compared to $5.4 million and $9.0 million for the three and six months ended June 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 six months ended June 30, 2019, the Company granted 1,748,488 RSUs with service-based vesting conditions with a weighted-average fair value of $92.69 per share.

During the six months ended June 30, 2019, the Company granted options to purchase 610,250 shares of common stock with a weighted-average fair value of $36.84 per share.

(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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Expected volatility	37.1 – 37.2%	38.4%	37.1 – 37.4%	37.8 – 38.4%
Dividend yield	0.0%	0.0%	0.0%	0.0%
Expected life (in years)	5.8	5.7	4.6 – 5.8	4.6 – 5.7
Risk-free interest rate	2.2 – 2.4%	2.7%	2.2 – 3.0%	2.3 – 2.7%

The Company issued 107,473 new stock purchase rights under the Employee Stock Purchase Plan during the six months ended June 30, 2019.

Restricted Stock Unit Awards with Performance Conditions

In March 2019, the Compensation Committee and Board of Directors (Board) approved the grant of 99,010 RSUs with performance-based vesting conditions (base RSUs) and a grant date fair value of $94.53. 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, dependent 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 RSUs, if any, vest 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 that 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. 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.

(16) INCOME TAXES

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. 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, resulting in a net tax benefit of $27.1 million recognized during the second quarter of 2019.

(17) NET LOSS PER COMMON SHARE

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

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):

	Three and Six Months Ended June 30,	
	2019	2018
Options to purchase common stock	7,494	7,829
Common stock issuable under the 2018 Notes	—	3,983
Common stock issuable under the 2020 Notes	3,983	3,983
Common stock issuable under the 2024 Notes	3,970	3,970
Unvested restricted stock units	4,108	3,544
Common stock potentially issuable for ESPP purchases	482	433
Common stock held by the NQDC	211	208
Total number of potentially issuable shares	20,248	23,950

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 June 30, 2019 as the Company's closing stock prices on June 28, 2019 (the last trading day before June 30, 2019) did not exceed the conversion price of $94.15 per share. Although the Company's stock price on June 29, 2018 (the last trading day before June 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 six months ended June 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 June 30, 2019, the Company was subject to contingent payments totaling approximately $391.1 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, $68.2 million relates to the acquisition of certain rights and other assets with respect to Kuvan and Palynziq from a third party and $241.1 million relates to programs that are no longer being developed.

As of June 30, 2019, the Company recorded a total of $70.3 million of contingent liabilities, of which $20.1 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 June 30, 2019, such commitments and other minimum contractual obligations for clinical and post-marketing services were estimated at approximately $118.3 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 June 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 June 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 [2]	PKU	Not applicable	Not applicable	Preclinical

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

(2) A gene therapy product candidate for the treatment of PKU. We plan to submit an investigational new drug application (IND) and/or a clinical trial application (CTA) for a gene therapy product for the treatment of PKU in the second half of 2019.

(In millions, except as otherwise disclosed)

Business Developments

We continued to grow our commercial business and advance our product candidate pipeline during the first half 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:

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 are expected to commence in the third quarter of 2019.

Continued Emphasis on Research and 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 the valoctocogene roxaparvovec based on recent meetings with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

  We also 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.
- 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 years. 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.
- We have announced plans to cease the development of BMN 290 program for Friedreich's Ataxia program based on progress of other portfolio assets that have demonstrated stronger product profiles.

Financial Highlights

Key components of our results of operations include the following:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Total revenues	$ 387.8	$ 372.8	$ 788.5	$ 746.3
Cost of sales	77.4	79.0	166.6	161.4
Research and development (R&D) expense	185.6	175.6	369.2	359.5
Selling, general and administrative (SG&A) expense	160.7	153.3	322.9	291.6
Intangible asset amortization and contingent consideration	20.3	10.2	40.1	23.4
Gain on sale of intangible assets	(15.0)	(20.0)	(15.0)	(20.0)
Net loss	(37.4)	(16.8)	(93.9)	(60.9)

The increase in Net Loss for the three months ended June 30, 2019 as compared to the three months ended June 30, 2018 was primarily due to the following:

- 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 roxaparvovec development program;
- 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; and
- increased sales and marketing expense primarily in support of the EU commercial launch and continued U.S. expansion of Palynziq and precommercialization activities related to valoctocogene roxaparvovec; partially offset by
- increased gross profit driven by increased sales revenue from products we market.

The increase in Net Loss for the six months ended June 30, 2019 as compared to the six months ended June 30, 2018 was primarily attributed to the following:

- increased sales and marketing expense primarily in support of the EU commercial launch and continued U.S. expansion of Palynziq and valoctocogene roxaparvovec pre-commercialization activities;
- 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; and
- increased R&D expense primarily attributed to preclinical and manufacturing activities related to our PKU gene therapy development program and clinical and manufacturing activities related to our valoctocogene roxaparvovec development program; partially offset by
- increased gross profit driven by increased sales revenue from products we market.

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

Our cash, cash equivalents and investments totaled approximately $1.1 billion as of June 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.

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 six months ended June 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.

(In millions, except as otherwise disclosed)

Results of Operations

Revenues

Net Product Revenues consisted of the following:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2019	2018	Change	2019	2018	Change
Aldurazyme	$ 5.8	$ 24.0	$ (18.2)	$ 51.1	$ 90.1	$ (39.0)
Brineura	14.8	10.9	3.9	27.0	17.8	9.2
Firdapse	5.5	5.2	0.3	10.6	10.1	0.5
Kuvan	113.3	109.0	4.3	220.2	208.1	12.1
Naglazyme	98.2	91.1	7.1	185.1	166.1	19.0
Palynziq	18.8	—	18.8	31.1	—	31.1
Vimizim	122.7	127.6	(4.9)	248.5	244.7	3.8
Total net product revenues	$ 379.1	$ 367.8	$ 11.3	$ 773.6	$ 736.9	$ 36.7

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 June 30, 2019 as compared to the three months ended June 30, 2018 was primarily attributed to the following:

- Palynziq: the increase was primarily attributed to new patients initiating therapy in the U.S. as the product launched in the third quarter of 2018;
- Naglazyme: the increase was primarily attributed to increased sales volume driven by government ordering patterns from certain Latin American and European countries; and
- Kuvan: the increase was primarily attributed to increased sales volume driven by government ordering patterns from certain European countries; partially offset by
- Aldurazyme: the decrease was primarily attributed to the unfavorable timing of customer acceptance for product shipped to Genzyme; and
- Vimizim: the decrease was primarily attributed to decreased sales volume driven by government ordering patterns in certain Latin American countries and European countries.

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

- Palynziq: the increase was primarily attributed to new patients initiating therapy in the U.S. as the product launched in the US during the third quarter of 2018;
- Naglazyme: the increase was primarily attributed to increased sales volume driven by government ordering patterns from certain Latin American and European countries;
- Kuvan: the increase was primarily attributed to increased sales volume driven by government ordering patterns from certain European countries and new patients initiating therapy in the U.S.; and
- Brineura: the increase was primarily attributed to increased sales volume driven by government ordering patterns in certain European countries and new patients initiating therapy; partially offset by
- Aldurazyme: the decrease was primarily attributed to the unfavorable 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.

(In millions, except as otherwise disclosed)

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2019	2018	Change	2019	2018	Change
Sales denominated in USD	$ 218.8	$ 218.9	$ (0.1)	$ 454.0	$ 455.3	$ (1.3)
Sales denominated in foreign currencies	160.3	148.9	11.4	319.6	281.6	38.0
Total Net Product Revenues	$ 379.1	$ 367.8	$ 11.3	$ 773.6	$ 736.9	$ 36.7

The net impact of foreign currency exchange rates on product sales denominated in currencies other than USD during the three months ended June 30, 2019 was negative by $6.4 million, compared to a positive impact of $3.4 million for the three months ended June 30, 2018, both of which were driven primarily by the fluctuations in Euro exchange rates. The net impact of foreign currency exchange rates on product sales denominated in currencies other than USD during the six months ended June 30, 2019 was negative by $11.7 million, compared to a positive impact of $9.5 million for the six months ended June 30, 2018, both of which were driven primarily by the fluctuations in Euro exchange rates.

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.

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2019	2018	Change	2019	2018	Change
Total Net Product Revenues	$ 379.1	$ 367.8	$ 11.3	$ 773.6	$ 736.9	$ 36.7
Cost of Sales	77.4	79.0	(1.6)	166.6	161.4	5.2
Product gross margin	80%	79%	1%	78%	78%	—%

Our Cost of Sales decreased for the three months ended June 30, 2019 compared to the same period in 2018 due to lower per-unit costs for Naglazyme and Vimizim, which resulted in improved product gross margins for the period. Our cost of sales for the six months ended June 30, 2019 compared to 2018 increased due to increased sales volumes and product gross margins 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. During the second quarter of 2019, we capitalized $1.9 million of manufacturing related costs for the commercial production of valoctocogene roxaparvovec as we believe those costs are recoverable. See Note 8 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our inventory.

(In millions, except as otherwise disclosed)

R&D expense consisted of the following:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2019	2018	Change	2019	2018	Change
Valoctocogene roxaparvovec	$ 50.9	$ 37.2	$ 13.7	$ 103.9	$ 72.2	$ 31.7
Vosoritide	28.1	19.5	8.6	60.1	40.4	19.7
Tralesinidase alfa	7.0	23.6	(16.6)	15.8	54.3	(38.5)
PKU gene therapy (BMN 307)	29.4	3.5	25.9	41.2	5.3	35.9
Palynziq	17.2	22.4	(5.2)	40.0	56.9	(16.9)
Brineura	10.7	12.8	(2.1)	21.2	24.2	(3.0)
Other approved products	16.9	17.7	(0.8)	34.0	35.6	(1.6)
Early stage programs	19.9	15.8	4.1	36.8	32.6	4.2
Other	5.5	23.1	(17.6)	16.2	38.0	(21.8)
Total	$ 185.6	$ 175.6	$ 10.0	$ 369.2	$ 359.5	$ 9.7

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

- an increase in preclinical and manufacturing activities related to our PKU gene therapy development program, including clinical manufacturing costs;
- an increase in clinical activity related to our late-stage product candidates valoctocogene roxaparvovec and vosoritide; partially offset by
- a decrease in other R&D expenses related to development activities that are not allocated to programs;
- a decrease in tralesinidase alfa clinical manufacturing costs; and
- a decrease in clinical manufacturing costs for Palynziq, which was approved in May 2018 at which time we began capitalizing manufacturing costs to inventory.

For the remainder of 2019, we expect our R&D spending to increase over 2018 levels primarily due to valoctocogene roxaparvovec, vosoritide, PKU gene therapy and other programs progressing in their development; partially offset by reduced clinical expenses for Palynziq. We also expect increased spending on preclinical activities for our early development stage programs. 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 June 30,			Six Months Ended June 30,		
	2019	2018	Change	2019	2018	Change
Selling & Marketing expense	$ 87.7	$ 81.1	$ 6.6	$ 171.2	$ 157.2	$ 14.0
General & Administrative expense	73.0	72.2	0.8	151.7	134.4	17.3
Total SG&A expense	$ 160.7	$ 153.3	$ 7.4	$ 322.9	$ 291.6	$ 31.3

	Three Months Ended June 30,			Six Months Ended June 30,		
Selling & Marketing expense by product	2019	2018	Change	2019	2018	Change
Palynziq	$ 15.5	$ 7.4	$ 8.1	$ 28.8	$ 12.4	$ 16.4
Brineura	10.3	10.6	(0.3)	19.7	21.1	(1.4)
Valoctocogene roxaparvovec	9.2	3.7	5.5	16.1	7.3	8.8
Other approved products	46.1	54.9	(8.8)	93.9	106.0	(12.1)
Other	6.6	4.5	2.1	12.7	10.4	2.3
Total Selling & Marketing expense	$ 87.7	$ 81.1	$ 6.6	$ 171.2	$ 157.2	$ 14.0

(In millions, except as otherwise disclosed)

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

- an increase in S&M expense in support of the continued U.S. and EU commercial launches of Palynziq and pre-commercialization activities relate to valoctocogene roxaparvovec; partially offset by
- a decrease commercial activities related to our other approved products; 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 and the PKU franchise.

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 June 30,			Six Months Ended June 30,		
	2019	2018	Change	2019	2018	Change
Changes in the fair value of contingent consideration	$ 6.9	$ 2.7	$ 4.2	$ 19.2	$ 8.3	$ 10.9
Amortization of intangible assets	13.4	7.5	5.9	20.9	15.1	5.8
Total intangible asset amortization and contingent consideration	$ 20.3	$ 10.2	$ 10.1	$ 40.1	$ 23.4	$ 16.7
Gain on sale of intangible assets	$ (15.0)	$ (20.0)	$ 5.0	$ (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 six months ended June 30, 2019 were attributable to changes in the estimated probability of achieving development 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 six months ended June 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 three and six months ended June 30, 2019 due to a third party's achievement of a milestone 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 June 30,			Six Months Ended June 30,		
	2019	2018	Change	2019	2018	Change
Interest income	$ 5.9	$ 5.6	$ 0.3	$ 12.2	$ 10.8	$ 1.4

The increase in interest income for the three and six months ended June 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 June 30,			Six Months Ended June 30,		
	2019	2018	Change	2019	2018	Change
Coupon interest expense	$ 2.4	$ 3.5	$ (1.1)	$ 4.7	$ 6.5	$ (1.8)
Amortization of issuance costs	0.5	1.0	(0.5)	1.0	2.0	(1.0)
Accretion of discount on convertible notes	4.0	7.7	(3.7)	7.9	15.3	(7.4)
Total interest expense	$ 6.9	$ 12.2	$ (5.3)	$ 13.6	$ 23.8	$ (10.2)

The decrease in Interest Expense for the three and six months ended June 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. We do not expect Interest Expense to fluctuate significantly over the next 12 months. 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 six months ended June 30, 2019, we recognized an income tax benefit of $4.5 million and $0.9 million, respectively, compared to the three and six months ended June 30, 2018 when we recognized an income tax benefit of $12.4 million and $19.0 million, respectively. U.S. and foreign tax expense was computed using a forecasted annual effective tax rate for the three and six months ended June 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 income tax benefit for the three and six months ended June 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 on foreign net operating loss carryforwards that resulted in a net income tax benefit in the current period. The income tax benefit for the three and six months ended June 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 six months ending June 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.

Financial Position, Liquidity and Capital Resources

As of June 30, 2019, we had approximately $1.1 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 June 30, 2019, $154.1 million of our $1.1 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.

(In millions, except as otherwise disclosed)

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

	June 30, 2019	December 31, 2018	Change
Cash and cash equivalents	$ 307.6	$ 494.0	$ (186.4)
Short-term investments	423.5	590.3	(166.8)
Long-term investments	375.0	235.9	139.1
Cash, cash equivalents and investments	$ 1,106.1	$ 1,320.2	$ (214.1)
Convertible debt	$ 839.2	$ 830.4	$ 8.8

Our cash flows for the six months ended June 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	(79.2)	(76.4)	(2.8)
Net cash used in investing activities	(30.8)	(67.0)	36.2
Net cash used in financing activities	(76.7)	(26.8)	(49.9)
Foreign exchange impact	0.3	(0.4)	0.7
Cash and cash equivalents at the end of the period	$ 307.6	$ 427.4	$ (119.8)
Short-term and long-term investments	798.5	1,215.7	(417.2)
Cash, cash equivalents and investments	$ 1,106.1	$ 1,643.1	$ (537.0)

Cash Used in Operating Activities

Cash used in operating activities increased by $2.8 million to $79.2 million in the six months ended June 30, 2019, compared to $76.4 million in the six months ended June 30, 2018. The increase is primarily attributed to higher inventory levels.

Cash Used in Investing Activities

Net cash used in investing activities decreased by $36.2 million to $30.8 million in the six months ended June 30, 2019, compared to $67.0 million during the six months ended June 30, 2018. The decrease in net cash used in investing activities during the six months ended June 30, 2019 was primarily attributable to the decrease in reinvestment of available-for-sale debt securities.

Cash Used in Financing Activities

Net cash used in financing activities increased by $49.9 million to $76.7 million used in the six months ended June 30, 2019, compared to $26.8 million during the six months ended June 30, 2018. The increase in net cash used in financing activities for the six months ended June 30, 2019 was primarily attributed to a decrease in proceeds from option exercises of $25.9 million, an increase in payments of contingent consideration of $14.4 million and an increase in cash paid for taxes related to the net settlement of equity awards of $8.3 million.

Other Information

Our $870.0 million (undiscounted) of total convertible debt as of June 30, 2019 will impact our liquidity due to the semi-annual cash interest payments. As of June 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 June 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;*
- *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 June 30, 2019 were as follows:

	Since Program Inception
Palynziq	$ 657.7
Valoctocogene roxaparvovec	504.9
Vosoritide	379.1
Brineura	308.3
PKU gene therapy	59.3
Other approved products	1,084.4
Other	Not meaningful

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 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; 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.

(In millions, except as otherwise disclosed)

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

	Payments Due Within				
	Remainder of 2019	>1 -3 Years	> 3 - 5 Years	More Than 5 Years	Total
2020 Notes and related interest	$ 2.8	$ 380.6	$ —	$ —	$ 383.4
2024 Notes and related interest	1.5	5.9	5.9	498.0	511.3
Leases	7.1	24.5	19.1	27.9	78.6
R&D and purchase commitments	110.1	8.2	—	—	118.3
Total	$ 121.5	$ 419.2	$ 25.0	$ 525.9	$ 1,091.6

We are also subject to contingent payments related to certain development and regulatory activities and commercial sales and licensing milestones totaling approximately $391.1 million as of June 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, $68.2 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 $241.1 million relates to programs that are no longer being developed.

As of June 30, 2019, we recorded $70.3 million of total contingent liabilities on our Condensed Consolidated Balance Sheets, of which $20.1 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 six months ended June 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 June 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.

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. 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 our product candidate 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.

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 June 30, 2019, we had cash, cash equivalents and investments totaling $1.1 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 June 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. 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 October 31, 2019, 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.

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 UK 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 order 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 provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction.

This exclusive-forum provision 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.

Exhibit Number	Description
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.
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.
10.1*†	First Amendment to the Amended and Restated BioMarin Pharmaceutical Inc. Nonqualified Deferred Compensation Plan, as adopted June 4, 2019
10.2*†	BioMarin Pharmaceutical Inc. Amended and Restated 2006 Employee Stock Purchase Plan, as amended and restated April 12, 2019
10.3*†	BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, as adopted April 10, 2017 and amended April 12, 2019
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 June 30, 2019, are embedded within the Inline XBRL document.

* Filed herewith

† Management contract or compensatory plan or arrangement

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

SIGNATURE

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

BIOMARIN PHARMACEUTICAL INC.

Dated: August 2, 2019

By /S/ DANIEL SPIEGELMAN

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

FIRST AMENDMENT TO
THE AMENDED AND RESTATED
BIOMARIN PHARMACEUTICAL INC. (the "Company")
NONQUALIFIED DEFERRED COMPENSATION PLAN (the "Plan")

(as amended and restated on October 7, 2014)

Effective Date: June 4, 2019

1. Subsection 1.26 of the Plan is amended and restated in its entirety to read as follows:

    1.26 **"Restricted Stock Compensation,"** means any restricted stock, restricted stock unit, phantom stock or similar award granted by the Employer to a Participant under any Employer-sponsored equity compensation plan and shares of Company stock resulting from the vesting of any such equity award.

2. The modifications set forth above shall not affect any other provisions of the Plan.

BioMarin Pharmaceutical Inc.
Amended and Restated 2006 Employee Stock Purchase Plan,
As Amended and Restated April 12, 2019

The following constitutes the provisions of the BioMarin Pharmaceutical Inc. Amended and Restated 2006 Employee Stock Purchase Plan of BioMarin Pharmaceutical Inc. (the "Company"), which is an amendment and restatement of the Company's 1998 Employee Stock Purchase Plan (which shall remain in full force and effect (including all Offering Periods, as defined below, in effect thereunder).

1. Purpose.

The purpose of the Plan is to provide employees of the Company and its Designated Subsidiaries with an opportunity to purchase Common Shares of the Company. It is the intention, but not the obligation, of the Company to have the Plan qualify as an "employee stock purchase plan" under Section 423 of the Code. The provisions of the Plan shall, accordingly, be construed so as to extend and limit participation in a manner consistent with the requirements of that section of the Code.

2. Definitions.

(a) *"**Administrator**"* means (i) any person, committee or Company department, division or function to whom the Board delegates administrative discretion under the Plan, and (ii) the Board, which may exercise any and all administrative powers associated with the Plan.

(b) *"**Board**"* means the Board of Directors of the Company.

(c) *"**Code**"* means the Internal Revenue Code of 1986, as amended.

(d) *"**Common Shares**"* means shares of common stock, par value $.001 per share, of the Company.

(e) *"**Company**"* means BioMarin Pharmaceutical Inc., a Delaware corporation.

(f) *"**Compensation**"* means the sums of the types and amounts of compensation determined from time to time by the Administrator in its sole discretion to be eligible to be taken into account under the Plan, provided that no such determination shall include or exclude any type or amount of compensation contrary to the requirements of Section 423 of the Code, including the equal treatment of participants having the same employer corporation.

(g) *"**Continuous Status as an Employee**"* means the absence of any interruption or termination of service as an Employee, Continuous Status as an Employee shall not be considered interrupted in the case of (i) sick leave; (ii) military leave; (iii) any other leave of absence approved by the Administrator, provided that such leave is for a period of not more than three months, unless reemployment upon the expiration of such leave is guaranteed by contract or statute, or unless provided otherwise pursuant to Company policy adopted from time to time; or (iv) in the case of transfers between locations of the Company or between the Company and its Designated Subsidiaries.

(h) *"**Contributions**"* means all amounts credited to the account of a participant pursuant to the Plan.

(i) *"**Corporate Transaction**"* means a sale of all or substantially all of the Company's assets, or a merger, consolidation, or other capital reorganization of the Company with or into another corporation, or any other transaction or series of related transactions in which the Company's stockholders immediately prior thereto own less than 50% of the voting shares of beneficial interest of the Company (or its successor or parent) immediately thereafter.

(j) *"**Designated Subsidiaries**"* means the Subsidiaries (or other entities with respect to sub-plans established under Section 19(d)) that have been designated by the Board from time to time in its sole discretion as eligible to participate in the Plan.

(k) *"**Employee**"* means any person, including an Officer, whom the Company or one of its Designated Subsidiaries classifies as an employee for payroll tax purposes.

(l) *"**Exchange Act**"* means the Securities Exchange Act of 1934, as amended.

(m) *"**Non-United States Offering**"* means a separate Offering Period covering eligible Employees of one or more Designated Subsidiaries, as described in Section 8(b) and 13(b).

(n) *"**Offering Date**"* means the first business day of each Offering Period (and shall thereby be the grant date for each Offering Period).

(o) *"**Offering Period**"* means a period of approximately twenty-four (24) months, commencing on the first business day on or after May 1 and November 1 of each year and terminating on the last business day of the periods ending twenty-four months later (or such other period that the Administrator may determine in its sole discretion before an Offering Date); provided that the first Offering Period under the Plan, as amended and restated herein, shall begin on May 1, 2005 and shall end on April 30, 2007.

(p) *"**Officer**"* means a person who is an officer of the Company within the meaning of Section 16 of the Exchange Act and the rules and regulations promulgated there under.

(q) *"**Plan**"* means this Amended and Restated 2006 Employee Stock Purchase Plan.

(r) *"**Purchase Date**"* means the last day of each Purchase Period of the Plan, provided, however, that if such date is not a business day, the "Purchase Date" shall mean the immediately preceding business day.

(s) *"**Purchase Period**"* means a period of six calendar months (or such other period of up to 27 consecutive months that the Administrator may determine in its sole discretion before an Offering Date), beginning on the day after each Purchase Date and ending on the next Purchase Date, except that the first Purchase Period of any Offering Period shall commence on the Enrollment Date and end with the next Purchase Date; provided, however, that the first Purchase Period under the Plan as amended and restated shall commence on May 1, 2006.

(t) *"**Purchase Price**"* means with respect to a Purchase Period an amount equal to 85% of the Fair Market Value (as defined in Section 7(b)) of a Share on the Offering Date or the Purchase Date, whichever is lower; provided, however, that the Administrator may before any Offering Date establish a different formula for determining the Purchase Price so long as the formula does not result in a lower Purchase Price than is allowable under Section 423(b)(6) of the Code.

(u) *"**Share**"* means one Common Share, as adjusted in accordance with Section 18.

(v) *"**Subsidiary**"* means a corporation (or an unincorporated entity of which the Company is a co-employer of its employees), domestic or foreign, of which not less than 50% of the voting shares are held by the Company or a Subsidiary, whether or not such corporation now exists or is hereafter organized or acquired by the Company or a Subsidiary.

3. Eligibility.

(a) Any person who is an Employee as of the date two (2) months before the Offering Date of a given Offering Period shall be eligible to participate in such Offering Period, subject to the requirements of Section 5(a) and the limitations imposed by Section 423(b) of the Code.

(b) Any provisions of the Plan to the contrary notwithstanding other than Section 3(c), no Employee shall be granted an option under the Plan (i) if, immediately after the grant, such Employee (or any other person whose stock would be attributed to such Employee pursuant to Section 424(d) of the Code) would own shares of beneficial ownership of the Company and/or hold outstanding options to purchase Shares possessing five percent (5%) or more of the total combined voting power or value of all classes of Shares of the Company or shares of common stock of any Subsidiary of the Company, or (ii) if such option would permit his or her rights to purchase Shares under all employee stock purchase plans (described in Section 423 of the Code) of the Company and its Subsidiaries to accrue at a rate that exceeds Twenty-Five Thousand Dollars ($25,000) of the Fair Market Value of such Shares (determined at the time such option is granted) for each calendar year in which such option is outstanding at any time.

(c) Employees of affiliates of the Company that are not corporate Subsidiaries, and Employees who are ineligible to participate pursuant to Section 3(b)(i) may, in the sole discretion of the Administrator, be eligible to participate in any Company sub-plan or sub-plans that the Administrator may establish in accordance with Section 19(d).

4. Offering Periods.

The Plan shall be implemented by consecutive, overlapping Offering Periods with a new Offering Period generally commencing on the first business day on or after May 1 or November 1 of each year (or on such other day as the Administrator shall determine), ending on the last day of the calendar month that is approximately twenty-four (24) months after the Purchase Period begins, and continuing thereafter until terminated in accordance with Section 20; provided, however, that the first Offering Period under the Plan shall be the Offering Period that commenced May 1, 2005. Notwithstanding any other provision in this Plan, in the event the Fair Market Value of a Share on the Offering Date of an ongoing Offering Period is greater than or equal to the Fair Market Value of a Share on the Offering Date of a newer Offering Period, then the ongoing Offering Period shall terminate and all participants (including those previously enrolled in ongoing Offering Periods) shall automatically become enrolled in the newer Offering Period. The Administrator shall have the power to change the duration and/or frequency of Offering Periods and Purchase

Periods with respect to future purchases without stockholder approval, provided that the Administrator shall communicate any such change to affected participants as soon as administratively practicable prior to the scheduled beginning of the first Purchase Period to be affected.

5. Participation.

(a) An eligible Employee may become a participant in the Plan by completing a subscription agreement and/or such other enrollment forms provided by the Company (the "Subscription Materials") and submitting such Subscription Materials with the Company and/or the stock brokerage or other financial services firms designated or approved by the Administrator from time to time (each, a "Designated Broker") prior to the date set by the Administrator applicable to an Offering Date. The Subscription Materials shall set forth the percentage of the participant's Compensation (subject to Section 6(a)) to be paid as Contributions pursuant to the Plan.

(b) Payroll deductions shall commence at the start of the first applicable payroll period following the Offering Date (or on such later date as may be determined and communicated to affected participants by the Administrator) and shall end on the last payroll paid on or prior to the last Purchase Period to which the Subscription Materials is applicable, unless sooner terminated by the participant as provided in Section 10.

(c) A participant's Subscription Materials shall remain in effect for successive Purchase Periods unless modified as provided in Section 6 or terminated as provided in Section 10.

6. Method of Payment of Contributions.

(a) Subject to the limitation set forth in Section 6(c), a participant shall elect to have payroll deductions made on each payday during the Purchase Period in an amount not less than one percent (1%) nor more than ten percent (10%) of such participant's Compensation on each payday during the Offering Period. All payroll deductions made by a participant shall be credited to his or her account under the Plan. A participant may not make any additional payments into such account. A participant's election of a certain payroll deduction rate shall automatically apply to subsequent Purchase Periods and Offering Periods unless modified by the participant within such time period prior to such Purchase Periods or Offering Periods as may be determined and communicated to participants by the Administrator.

(b) A participant may discontinue his or her participation in the Plan as provided in Section 10, and may increase or decrease the rate of his or her Contributions with respect to the current Purchase Period, subsequent Purchase Periods, or a subsequent Offering Period only in accordance with rules that the Administrator establishes before the Offering begins. Any change in rate shall be effective as soon as administratively practicable.

(c) Notwithstanding the foregoing, to the extent necessary to comply with Section 423(b)(8) of the Code and Section 3(b), the Administrator may reduce a participant's payroll deductions or terminate a participant's payroll deductions during any Purchase Period scheduled to end during the current calendar year. In such case, payroll deductions shall re-commence at the beginning of the first Purchase Period that is scheduled to end in the following calendar year at the rate last elected by such participant, unless the participant has withdrawn from the Purchase Period pursuant to Section 10.

7. Grant of Option.

(a) On the Offering Date of each Offering Period, each eligible Employee participating in such Offering Period shall be granted an option to purchase on each Purchase Date for each Purchase Period within the Offering Period a number of Shares determined by dividing such Employee's Contributions accumulated during such Purchase Period and retained in the participant's account as of the Purchase Date by the applicable Purchase Price; provided however that the maximum number of Shares an Employee may purchase during each Purchase Period shall not exceed 5,000 Shares (subject to adjustment pursuant to Section 18), and provided further that such purchase shall be subject to the limitations set forth in Sections 3(b) and 12. The Board may, for future Offering Periods, increase or decrease, in its absolute discretion, the maximum number of shares of the Company's Common Stock that an eligible Employee may purchase during each Purchase Period of such Offering Period. Exercise of the option shall occur as provided in Section 8, unless the participant has withdrawn from the Purchase Period pursuant to Section 10. The option shall expire on the last day of the Offering Period.

(b) The fair market value of the Company's Common Shares on a given date (the "Fair Market Value") shall be –

(i) the closing sales price of the Common Shares for such date (or, in the event that the Common Shares are not traded on such date, on the immediately preceding trading date), as reported by the New York Stock Exchange or the American Stock Exchange, or, if such price is not reported, then on the nearest preceding trading day during which a sale occurred; or

(ii) if such stock is not traded on either exchange but is quoted on NASDAQ or a successor quotation system (A) the last sales price (if the stock is then listed as a National Market Issue under The Nasdaq National Market System or any successor system) or (B) the mean of the bid and asked prices per-share of the Common Shares as reported by the NASDAQ or successor; or

(iii) in the event the Common Shares are not listed on a stock exchange or quoted on NASDAQ but is otherwise traded in the over-the-counter market, the Fair Market Value per share shall be the mean between the most recent representative bid and asked prices; or

(iv) if subsections (i)-(iii) do not apply, the fair market value established in good faith by the Board.

8. Exercise of Option.

(a) Unless a participant withdraws from a Purchase Period as provided in Section 10, his or her option for the purchase of Shares will be exercised automatically on each Purchase Date of a Purchase Period, and the maximum number of full Shares subject to the option will be purchased at the applicable Purchase Price with the accumulated Contributions in his or her account. No fractional Shares shall be sold or issued pursuant to the Plan. Any payroll deductions accumulated in a participant's account that are not sufficient to purchase a full Share shall be retained in the participant's account for the subsequent Purchase Period, subject to earlier withdrawal by the participant as provided in Section 10. Any other amounts left over in a participant's account after a Purchase Date shall be returned to the participant. The Shares purchased upon exercise of an option hereunder shall be deemed to be transferred to the participant on the Purchase Date. During his or her lifetime, a participant's option to purchase Shares hereunder is exercisable only by him or her.

(b) Where payroll deductions on behalf of participants who are citizens or residents of countries other than the United States (without regard to whether they are also citizens of the United States or resident aliens) are prohibited by applicable law, the Administrator may establish a Non-United States Offering covering all eligible Employees of one or more Designated Subsidiaries subject to such prohibition on payroll deductions. The Non-United States Offering shall provide another method for payment of the Purchase Price with such terms and conditions as shall be administratively convenient and comply with applicable law. On each Purchase Date of the Offering Period applicable to a Non-United States Offering, each participant who has not withdrawn from the Purchase Period pursuant to Section 10 and whose participation in such Offering Period has not otherwise terminated before such Purchase Date shall automatically acquire pursuant to the exercise of the participant's option a number of whole Shares determined in accordance with Section 8(a) to the extent of the total amount of the participant's plan account balance accumulated during the Offering Period in accordance with the method established by the Administrator and not previously applied toward the purchase of Shares. However, in no event shall the number of Shares purchased by a participant during such Offering Period exceed the number of Shares subject to the participant's option. The Company shall return to a participant in a Non-United States Offering in accordance with Section 8(a) any excess payroll deductions received from such participant.

9. Delivery.

As promptly as practicable after each Purchase Date of each Purchase Period, the number of Shares purchased by each participant upon exercise of his or her option shall be deposited into an account established in the participant's name with a Designated Broker.

10. Voluntary Withdrawal; Termination of Employment.

(a) Subject to applicable securities law restrictions (e.g., the Company's insider trading policy), a participant may withdraw all but not less than all the Contributions credited to his or her account under the Plan at any time prior to each Purchase Date by giving the Company or the Designated Broker a notice of withdrawal in the form or manner designated by the Company or the Designated Broker (the "Notice of Withdrawal") prior to the date determined and communicated by the Administrator. All of the participant's Contributions credited to his or her account will be paid to him or her promptly after receipt of his or her Notice of Withdrawal, no further Contributions for the purchase of Shares will be made during such Purchase Period, and his or her option will be automatically terminated.

(b) Upon termination of the participant's Continuous Status as an Employee prior to the Purchase Date of a Purchase Period for any reason, including retirement or death, the Contributions credited to his or her account will be returned to him or her or, in the case of his or her death, to the person or persons entitled thereto under Section 14, and his or her option will be automatically terminated.

(c) In the event an Employee fails to remain in Continuous Status as an Employee of the Company during the Purchase Period in which the employee is a participant, he or she will be deemed to have elected to withdraw from such Purchase Period and the Contributions credited to his or her account will be returned to him or her and his or her option will be automatically terminated.

(d) If a participant withdraws from a Purchase Period, the participant may enroll as a new participant in a subsequent Offering Period for which such participant is otherwise eligible by completing and submitting Subscription Materials pursuant to Section 5(a).

11. Interest.

No interest shall accrue on the Contributions of a participant in the Plan.

12. Shares.

(a) Subject to Section 18, the maximum number of Shares that participants may purchase pursuant to the Plan for Offering Periods beginning after on or after May 1, 2005 shall be seven million (7,000,000) Shares. The Shares that participants purchase pursuant to the Plan shall by newly-issued Shares, treasury Shares, or Shares purchased by the Designated Broker on the open market provided, however, that no more than seven million (7,000,000) Shares, (as adjusted pursuant to Section 18) shall be purchased pursuant to options under the Plan. In the latter case, to the extent the Purchase Price for Shares is below their Fair Market Value for any Purchase Period, the Company shall pay the Designated Broker such amounts as are necessary to subsidize the Purchase Price for Shares purchased on the open market.

(b) The participant shall have no interest (including no right to receive any dividends) or voting right in Shares covered by his or her option until such option has been exercised.

(c) Shares to be delivered to a participant under the Plan will be registered in the name of the participant or, if directed by the participant in writing, in the name of the participant and his or her spouse.

13. Administration.

(a) The Administrator shall supervise and administer the Plan, and shall have full and exclusive discretionary authority to construe, interpret, and apply the terms of the Plan, to determine eligibility, to adjudicate all disputed claims under the Plan, to adopt, amend and rescind any rules deemed appropriate for the administration of the Plan, and to make all other determinations necessary or advisable for the administration of the Plan. Every finding, decision, and determination made by the Administrator shall, to the full extent permitted by law, be final and binding upon all parties. No person acting individually or jointly as the Administrator shall be liable for any action or determination made in good faith with respect to the Plan or any participant.

(b) The Administrator shall have the power, in its discretion, to adopt one or more sub-plans of the Plan as the Administrator deems necessary or desirable to comply with the laws or regulations, tax policy, accounting principles or custom of foreign jurisdictions applicable to employees of a Subsidiary business entity of the Company, provided that any such sub-plan shall not be within the scope of an “employee stock purchase plan” within the meaning of Section 423 of the Code. Any of the provisions of any such sub-plan may supersede the provisions of this Plan, other than Section 12. Except as superseded by the provisions of a sub-plan, the provisions of this Plan shall govern such sub-plan. Alternatively and in order to comply with the laws of a foreign jurisdiction, the Administrator shall have the power, in its discretion, to grant options to citizens or residents of a non-U.S. jurisdiction (without regard to whether they are also citizens of the United States or resident aliens) that provide terms which are less favorable than or different from the terms of an option granted under the same Offering Period to Employees resident in the United States.

(c) Power to Establish Separate Offerings with Varying Terms. The Administrator shall have the power, in its discretion, to establish separate, simultaneous or overlapping Offering Periods having different terms and conditions and to designate the Designated Subsidiary or Subsidiaries that may participate in a particular Offering Period, provided that each Offering Period shall individually comply with the terms of the Plan and the requirements of Section 423(b)(5) of the Code that all participants granted options pursuant to such Offering Period shall have the same rights and privileges within the meaning of such section.

14. Designation of Beneficiary.

(a) A participant may designate a beneficiary who is to receive any Shares and cash, if any, from the participant’s account under the Plan in the event of such participant’s death subsequent to the end of a Purchase Period but prior to delivery to him or her of such Shares and cash. In addition, a participant may designate a beneficiary who is to receive any cash from the Participant’s account under the Plan in the event of such participant’s death prior to the Purchase Date. If a participant is married and the designated beneficiary is not the spouse, spousal consent shall be required for such designation to be effective. Beneficiary designations under this Section 14(a) shall be made in the form and in the manner as directed by the Company.

(b) Such designation of beneficiary may be changed by the participant (and his or her spouse, if any) at any time by written notice in accordance with Section 14(a). In the event of the death of a participant and in the absence of a

beneficiary validly designated under the Plan who is living at the time of such participant's death, the Company shall deliver such Shares and/or cash to the executor or administrator of the estate of the participant, or if no such executor or administrator has been appointed (to the knowledge of the Company), the Company, in its discretion, may deliver such Shares and/or cash to the spouse or to any one or more dependents or relatives of the participant, or if no spouse, dependent or relative is known to the Company, then to such other person as the Company may designate.

15. **Transferability.**

Neither Contributions credited to a participant's account nor any rights with regard to the exercise of an option or to receive Shares under the Plan may be assigned, transferred, pledged or otherwise disposed of in any way (other than by will, the laws of descent and distribution, or as provided in Section 14) by the participant. Any such attempt at assignment, transfer, pledge or other disposition shall be without effect, except that the Company may treat such act as an election to withdraw from a Purchase Period in accordance with Section 10.

16. Use of Funds.

All Contributions received or held by the Company under the Plan may be used by the Company for any corporate purpose, and the Company shall not be obligated to segregate such Contributions.

17. Reports.

Individual recordkeeping accounts will be maintained for each participant in the Plan. Statements of account will be provided to participating Employees at least annually by the Designated Broker, which statements will set forth the amounts of Contributions, the per Share Purchase Price, the number of Shares purchased, and the remaining cash balance, if any.

18. Adjustments Upon Corporate Transactions.

(a) In the event of a proposed dissolution or liquidation of the Company, any Purchase Period then in progress will terminate immediately prior to the consummation of such action, unless otherwise provided by the Board. In the event of a Corporate Transaction, each option outstanding under the Plan shall be assumed or an equivalent option shall be substituted by the successor corporation or a parent or Subsidiary of such successor corporation. In the event that the successor corporation refuses to assume or substitute for outstanding options, each Purchase Period then in progress shall be shortened and a new Purchase Date shall be set (the "New Purchase Date"), as of which date any Purchase Period then in progress will terminate. The New Purchase Date shall be on or before the date of consummation of the transaction and the Board shall provide reasonable notice to each participant in writing prior to the New Purchase Date, that the Purchase Date for his or her option has been changed to the New Purchase Date and that his or her option will be exercised automatically on the New Purchase Date, unless prior to such date he or she has withdrawn from the Purchase Period as provided in Section 10.

(b) For purposes of this Section 18, an option granted under the Plan shall be deemed to be assumed, without limitation, if, at the time of issuance of the stock or other consideration upon a Corporate Transaction, each holder of an option under the Plan would be entitled to receive upon exercise of the option the same number and kind of shares of stock or the same amount of property, cash or securities as such holder would have been entitled to receive upon the occurrence of the transaction if the holder had been, immediately prior to the transaction, the holder of the number of Shares covered by the option at such time (after giving effect to any adjustments in the number of Shares covered by the option as provided for in this Section 18); provided, however, that if the consideration received in the transaction is not solely common stock of the successor corporation or its parent (as defined in Section 424(e) of the Code), the Board may, with the consent of the successor corporation, provide for the consideration to be received upon exercise of the option to be solely common stock of the successor corporation or its parent equal in Fair Market Value to the per Share consideration received by holders of Common Shares in the transaction.

(c) The Administrator shall equitably adjust the number of Shares covered by each outstanding option, and the number of Shares that may be purchased pursuant to options under the Plan, as well as the price per Share covered by each such outstanding option, to reflect any increase or decrease in the number of issued Shares resulting from a stock-split, reverse stock-split, stock dividend, combination, recapitalization or reclassification of the Shares, or any other increase or decrease in the number of issued Shares effected without receipt of consideration by the Company. Except as expressly provided herein, no issuance by the Company of shares of stock of any class, or securities convertible into shares of stock of any class, shall affect, and no adjustment by reason thereof shall be required to be made with respect to, the number or price of Shares subject to any option.

19. Amendment or Termination.

(a) The Board may at any time and for any reason terminate or amend the Plan. Except as provided in Section 18, no such termination of the Plan may affect options previously granted, provided that the Plan or a

Purchase Period may be terminated by the Board on a Purchase Date or by the Board's setting a new Purchase Date with respect to a Purchase Period then in progress if the Board determines that termination of the Plan and/or the Purchase Period is in the best interests of the Company and the stockholders, or if continuation of the Plan and/or the Purchase Period would cause the Company to incur adverse accounting charges as a result of a change after the effective date of the Plan in the generally accepted accounting rules applicable to the Plan. Except as provided in Section 18 and in this Section 19, no amendment to the Plan shall make any change in any option previously granted that adversely affects the rights of any participant. In addition, to the extent the Administrator considers it appropriate to conform the Plan with Rule 16b-3 under the Exchange Act, Section 423 of the Code, or any other applicable law, regulation, or stock exchange rule, the Company shall obtain stockholder approval in such a manner and to such a degree as so required.

(b) Without stockholder consent and without regard to whether any participant rights may be considered to have been adversely affected, the Board (or its committee) shall be entitled to change the Purchase Periods, to limit the frequency and/or number of changes in the amount withheld during a Purchase Period, to establish the exchange ratio applicable to amounts withheld in a currency other than U.S. dollars, to permit payroll withholding in excess of the amount designated by a participant in order to adjust for delays or mistakes in the Company's processing of properly completed withholding elections, to establish reasonable waiting and adjustment periods and/or accounting and crediting procedures to ensure that amounts applied toward the purchase of Common Shares for each participant properly correspond with amounts withheld from the participant's Compensation, and to establish such other limitations or procedures as the Board (or its committee) determines in its sole discretion advisable that are consistent with the Plan.

(c) The Company may adopt rules or procedures relating to the operation and administration of the Plan to accommodate the specific requirements of local laws and procedures. Without limiting the generality of the foregoing, the Company specifically authorizes the Administrator to adopt rules and procedures regarding handling of payroll deductions, payment of interest, conversion of local currency, payroll tax, withholding procedures and handling of stock certificates which vary with local requirements.

(d) The Administrator may also adopt sub-plans applicable to the Company or to particular Subsidiaries, or locations, which sub-plans may be designed to be outside the scope of Section 423 of the Code. The rules of such sub-plans may take precedence over other provisions of this Plan, but unless otherwise superseded by the specific terms of such sub-plan, the provisions of this Plan shall govern the operation of such sub-plan. In addition, the Administrator may adopt rules or procedures relating to the operation and administration of the Plan to accommodate the specific requirements of local laws and procedures. Without limiting the generality of the foregoing, the Company is specifically authorized to adopt rules and procedures regarding handling of payroll deductions, payment of interest, conversion of local currency, payroll tax, withholding procedures and handling of stock certificates which vary with local requirements.

20. Notices.

All notices or other communications by a participant to the Company under or in connection with the Plan shall be deemed to have been duly given when received in the form specified by the Company at the location, or by the person, designated by the Company for the receipt thereof.

21. Conditions Upon Issuance of Shares.

Shares shall not be issued with respect to an option unless the exercise of such option and the issuance and delivery of such Shares pursuant thereto shall comply with all applicable provisions of law, domestic or foreign, including, without limitation, the Securities Act of 1933, as amended, the Exchange Act, the rules and regulations promulgated thereunder, applicable state securities laws, and the requirements of any stock exchange upon which the Shares may

then be listed, and shall be further subject to the approval of counsel for the Company with respect to such compliance.

As a condition to the exercise of an option, the Company may require the person exercising such option to represent and warrant at the time of any such exercise that the Shares are being purchased only for investment and without any present intention to sell or distribute such Shares if, in the opinion of counsel for the Company, such a representation is required by any of the aforementioned applicable provisions of law.

22. Term of Plan; Effective Date.

The Plan, as amended and restated herein, shall become effective on April 12, 2019, provided that the effectiveness of the Plan, as amended and restated herein, shall be contingent on the Company's receipt of approval in by a vote of a majority of the votes cast at a duly held meeting of the Company's stockholders (or by such other stockholder vote that the Administrator determines to be sufficient for the issuance of Shares or stock options according to the Company's governing documents and applicable state law). The Plan shall continue in effect until terminated under Section 19. In the event that the Plan, as amended and restated herein, does not receive stockholder approval, then the Plan, as amended and restated herein, shall not become effective and the Plan as in effect prior to this amendment and restatement will continue in full force and effect and continue to apply to ongoing Offering Periods that were in effect prior to the Plan's termination on May 2, 2018.

23. Additional Restrictions of Rule 16b-3.

The terms and conditions of options granted hereunder to, and the purchase of Shares by, persons subject to Section 16 of the Exchange Act shall comply with the applicable provisions of Rule 16b-3. This Plan shall be deemed to contain, and such options shall contain, and the Shares issued upon exercise thereof shall be subject to, such additional conditions and restrictions as may be required by Rule 16b-3 to qualify for the maximum exemption from Section 16 of the Exchange Act with respect to Plan transactions.

24. Notice of Disqualifying Dispositions.

By electing to participate in the Plan, each participant agrees to notify the Company in writing immediately after the participant sells, transfers or otherwise disposes of any Shares acquired under the Plan, if such disposition occurs within the earlier of (i) two (2) years of the Offering Date, or (ii) one (1) year of the Purchase Date, associated with such Shares. Each participant further agrees to provide any information about a disposition of Shares as may be requested by the Company to assist it in complying with any applicable tax laws.

25. Withholding of Taxes.

Each participant must make adequate provision for all applicable federal, state, or other tax withholding obligations which may arise upon the exercise of any option or the disposition of any Shares.

26. No Employment Rights.

The Plan does not create, directly or indirectly, any right for the benefit of any employee or class of employees to purchase any Shares from the Company (other than as expressly provided in, and subject to the terms and conditions of, the Plan), or create in any employee or class of employees any right with respect to continuation of employment by the Company or any Subsidiary, and it shall not be deemed to interfere in any way with the Company's or any Subsidiary's right to terminate, or otherwise modify, an employee's employment at any time.

27. Offsets.

To the extent permitted by law, the Company shall have the absolute right to withhold any amounts payable to any participant under the terms of the Plan to the extent of any amount owed for any reason by such participant to the Company or any Subsidiary and to set off and apply the amounts so withheld to payment of any such amount owed to the Company or any Subsidiary, whether or not such amount shall then be immediately due and payable and in such order or priority as among such amounts owed as the Board or its committee, in its sole discretion, shall determine.

28. Captions.

The captions of the sections and paragraphs of this Plan have been inserted solely as a matter of convenience and in no way define or limit the scope or intent of any provision of the Plan. References to sections herein are to the specified sections of this Plan unless another reference is specifically stated. Wherever used herein, a singular number shall be deemed to include the plural unless a different meaning is required by the context.

29. Governing Law.

The internal laws of the State of Delaware shall govern all matters relating to this Plan except to the extent superseded by the laws of the United States.

BioMarin Pharmaceutical Inc.
2017 Equity Incentive Plan, As Amended April 12, 2019

Adopted by the Compensation Committee of the Board of Directors: April 10, 2017
Approved by the Stockholders: June 6, 2017

Amended by the Compensation Committee of the Board of Directors: April 12, 2019
Amendment Approved by the Stockholders: June 4, 2019

1. General.

(a) Successor to and Continuation of Prior Plan. The Plan is intended as the successor to and continuation of the Company's 2006 Share Incentive Plan, as amended and restated on April 16, 2015 (the "***2006 Plan***"). From and after 12:01 a.m. Pacific Time on the Effective Date, no additional awards will be granted under the 2006 Plan. All Awards granted on or after 12:01 a.m. Pacific Time on the Effective Date will be granted under this Plan. All awards granted under the 2006 Plan or under the Company's 1997 Stock Plan or the Company's 1998 Director Option Plan (collectively, with the 2006 Plan, the "***Prior Plans***"), will remain subject to the terms of the Prior Plans.

(i) Any shares that would otherwise remain available for future grants under the 2006 Plan as of 12:01 a.m. Pacific Time on the Effective Date (the "***2006 Plan's Available Reserve***") will cease to be available under the 2006 Plan at such time. Instead, that number of shares of Common Stock equal to the 2006 Plan's Available Reserve will be added to the Share Reserve (as further described in Section 3(a) below) and will be immediately available for grants and issuance pursuant to Stock Awards hereunder, up to the maximum number set forth in Section 3(a) below.

(ii) In addition, from and after 12:01 a.m. Pacific Time on the Effective Date, any shares subject, at such time, to outstanding stock awards granted under the 2006 Plan that (i) expire or terminate for any reason prior to exercise or settlement; or (ii) are forfeited because of the failure to meet a contingency or condition required to vest such shares (such shares the "***Returning Shares***") will immediately be added to the Share Reserve (as further described in Section 3(a) below) as and when such shares become Returning Shares, up to the maximum number set forth in Section 3(a) below.

(b) Eligible Award Recipients. Employees, Directors and Consultants are eligible to receive Awards.

(c) Available Awards. The Plan provides for the grant of the following Awards: (i) Incentive Stock Options, (ii) Nonstatutory Stock Options, (iii) Stock Appreciation Rights, (iv) Restricted Stock Awards, (v) Restricted Stock Unit Awards, (vi) Performance Stock Awards, (vii) Performance Cash Awards, and (viii) Other Stock Awards.

(d) Purpose. The Plan, through the grant of Awards, is intended to help the Company secure and retain the services of eligible award recipients, provide incentives for such persons to exert maximum efforts for the success of the Company and any Affiliate, and provide a means by which the eligible recipients may benefit from increases in value of the Common Stock.

2. Administration.

(a) Administration by Board. The Board will administer the Plan. The Board may delegate administration of the Plan to a Committee or Committees, as provided in Section 2(c).

(b) Powers of Board. The Board will have the power, subject to, and within the limitations of, the express provisions of the Plan:

(i) To determine: (A) who will be granted Awards; (B) when and how each Award will be granted; (C) what type of Award will be granted; (D) the provisions of each Award (which need not be identical), including when a person will be permitted to exercise or otherwise receive cash or Common Stock under the Award; (E) the number of shares of Common Stock subject to, or the cash value of, an Award; and (F) the Fair Market Value applicable to a Stock Award.

(ii) To construe and interpret the Plan and Awards granted under it, and to establish, amend and revoke rules and regulations for administration of the Plan and Awards. The Board, in the exercise of these powers, may correct any defect, omission or inconsistency in the Plan or in any Award Agreement or in the written terms of a Performance Cash Award, in a manner and to the extent it will deem necessary or expedient to make the Plan or Award fully effective.

(iii) To settle all controversies regarding the Plan and Awards granted under it.

(iv) To accelerate, in whole or in part, the time at which an Award may be exercised or vest (or the time at which cash or shares of Common Stock may be issued in settlement thereof).

(v) To suspend or terminate the Plan at any time. Except as otherwise provided in the Plan or an Award Agreement, suspension or termination of the Plan will not materially impair a Participant's rights under the Participant's then-outstanding Award without the Participant's written consent, except as provided in subsection (viii) below.

(vi) To amend the Plan in any respect the Board deems necessary or advisable, including, without limitation, by adopting amendments relating to Incentive Stock Options and certain nonqualified deferred compensation under Section 409A of the Code and/or bringing the Plan or Awards granted under the Plan into compliance with the requirements for Incentive Stock Options or ensuring that they are exempt from, or compliant with, the requirements for nonqualified deferred compensation under Section 409A of the Code, subject to the limitations, if any, of applicable law. If required by applicable law or listing requirements, and except as provided in Section 9(a) relating to Capitalization Adjustments, the Company will seek shareholder approval of any amendment of the Plan that (A) materially increases the number of shares of Common Stock available for issuance under the Plan, (B) materially expands the class of individuals eligible to receive Awards under the Plan, (C) materially increases the benefits accruing to Participants under the Plan, (D) materially reduces the price at which shares of Common Stock may be issued or purchased under the Plan, (E) materially extends the term of the Plan, or (F) materially expands the types of Awards available for issuance under the Plan. Except as otherwise provided in the Plan or an Award Agreement, no amendment of the Plan will materially impair a Participant's rights under an outstanding Award without the Participant's written consent.

(vii) To submit any amendment to the Plan for shareholder approval, including, but not limited to, amendments to the Plan intended to satisfy the requirements of (A) Section 162(m) of the Code regarding the exclusion of performance-based compensation from the limit on corporate deductibility of compensation paid to Covered Employees, (B) Section 422 of the Code regarding "incentive stock options" or (C) Rule 16b-3.

(viii) To approve forms of Award Agreements for use under the Plan and to amend the terms of any one or more Awards, including, but not limited to, amendments to provide terms more favorable to the Participant than previously provided in the Award Agreement, subject to any specified limits in the Plan that are not subject to Board discretion (including, without limitation, the limits set forth in Sections 8(c) and 8(m) below); *provided, however,* that a Participant's rights under any Award will not be impaired by any such amendment unless (A) the Company requests the consent of the affected Participant, and (B) such Participant consents in writing. Notwithstanding the foregoing, (1) a Participant's rights will not be deemed to have been impaired by any such amendment if the Board, in its sole discretion, determines that the amendment, taken as a whole, does not materially impair the Participant's rights, and (2) subject to the limitations of applicable law, if any, the Board may amend the terms of any one or more Awards without the affected Participant's consent (A) to maintain the qualified status of the Award as an Incentive Stock Option under Section 422 of the Code; (B) to change the terms of an Incentive Stock Option, if such change results in impairment of the Award solely because it impairs the qualified status of the Award as an Incentive Stock Option under Section 422 of the Code; (C) to clarify the manner of exemption from, or to bring the Award into compliance with, Section 409A of the Code; or (D) to comply with other applicable laws or listing requirements.

(ix) Generally, to exercise such powers and to perform such acts as the Board deems necessary or expedient to promote the best interests of the Company and that are not in conflict with the provisions of the Plan or Awards.

(x) To adopt such procedures and sub-plans as are necessary or appropriate to permit participation in the Plan by Employees, Directors or Consultants who are foreign nationals or employed outside the United States (provided that Board approval will not be necessary for immaterial modifications to the Plan or any Award Agreement that are required for compliance with the laws of the relevant foreign jurisdiction).

(c) Delegation to Committee.

(i) General. The Board may delegate some or all of the administration of the Plan to a Committee or Committees. If administration of the Plan is delegated to a Committee, the Committee will have, in connection with the administration of the Plan, the powers theretofore possessed by the Board that have been delegated to the Committee, including the power to delegate to a subcommittee of the Committee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board will thereafter be to the Committee or subcommittee, as applicable). Any delegation of administrative powers will be reflected in resolutions, not inconsistent

with the provisions of the Plan, adopted from time to time by the Board or Committee (as applicable). The Board may retain the authority to concurrently administer the Plan with the Committee and may, at any time, revest in the Board some or all of the powers previously delegated.

(ii) Section 162(m) and Rule 16b-3 Compliance. The Committee shall consist solely of two or more directors that qualify as Outside Directors, in accordance with Section 162(m) of the Code, and Non-Employee Directors, in accordance with Rule 16b-3.

(d) Delegation to an Officer. To the extent permissible under applicable law, the Board may delegate to one (1) or more Officers the authority to do one or both of the following (i) designate Employees who are not Officers to be recipients of Options and SARs (and, to the extent permitted by applicable law, other Stock Awards) and, to the extent permitted by applicable law, the terms of such Awards, and (ii) determine the number of shares of Common Stock to be subject to such Stock Awards granted to such Employees; *provided, however,* that the Board resolutions regarding such delegation will specify the total number of shares of Common Stock that may be subject to the Stock Awards granted by such Officer and that such Officer may not grant a Stock Award to himself or herself. Any such Stock Awards will be granted on the form of Stock Award Agreement most recently approved for use by the Committee or the Board, unless otherwise provided in the resolutions approving the delegation authority. The Board may not delegate authority to an Officer who is acting solely in the capacity of an Officer (and not also as a Director) to determine the Fair Market Value pursuant to Section 13(y)(iii) below.

(e) Effect of Board's Decision. All determinations, interpretations and constructions made by the Board in good faith will not be subject to review by any person and will be final, binding and conclusive on all persons.

(f) No Repricing of Awards. Neither the Board nor any Committee will have the authority to (i) reduce the exercise or strike price of any outstanding Option or SAR or (ii) cancel any outstanding Option or SAR that has an exercise or strike price (per share) greater than the then-current Fair Market Value of the Common Stock in exchange for cash or other Stock Awards under the Plan, unless the stockholders of the Company have approved such an action within twelve (12) months prior to such an event.

3. Shares Subject to the Plan.

(a) Share Reserve. Subject to Section 9(a) relating to Capitalization Adjustments, the aggregate number of shares of Common Stock that may be issued pursuant to Stock Awards will not exceed 31,880,015 shares (the "***Share Reserve***"), which number is the sum of (i) 11,000,000 new shares, *plus* (ii) 5,250,000 shares approved by the stockholders on June 6, 2017, plus (iii) the number of shares subject to the 2006 Plan's Available Reserve, *plus* (iv) the number of shares that are Returning Shares, as such shares become available from time to time (in the case of (iii) and (iv), up to an aggregate maximum of 15,630,015 shares). For every one share of Common Stock that is subject to a Stock Award other than an Option or SAR, the shares available for issuance under the Plan shall be reduced by 1.92 shares. For every one share of Common Stock that is subject to an Option or SAR, the shares available for issuance under the Plan shall be reduced by one share. The issuance of Substitute Awards will not reduce the number of shares available for issuance under the Plan.

(b) Reversion of Shares to the Share Reserve.

(i) Shares Available for Subsequent Issuance. The following shares of Common Stock will become available again for issuance under the Plan: (A) any shares subject to a Stock Award that are not issued because such Stock Award or any portion thereof expires or otherwise terminates without all of the shares covered by such Stock Award having been issued; (B) any shares issued pursuant to a Stock Award that are forfeited back to or repurchased by the Company because of the failure to meet a contingency or condition required for the vesting of such shares. Any shares that again become available for issuance pursuant to this paragraph shall be added back as (a) one (1) share for every one (1) share that is subject to an Award granted under the 2006 Plan prior to May 12, 2010; (b) one (1) share for every one (1) share that is subject to an Option granted under the 2006 Plan on or after May 12, 2010; (c) 1.62 shares for every one (1) share that is subject to any Award granted under the 2006 Plan on or after May 12, 2010 and prior to May 15, 2013 other than an Option; (d) 1.92 shares for every one (1) share that is subject to any Award granted under the 2006 Plan on or after May 15, 2013 other than an Option; (e) one (1) share for every one (1) share that is subject to an Option or SAR granted under this Plan; and (f) 1.92 Shares for every one (1) share that is subject to an Award granted under this Plan other than an Option or SAR.

(ii) Shares Not Available for Subsequent Issuance. The following shares of Common Stock will not become available again for issuance under the Plan: (A) any shares that are reacquired or withheld (or not issued) by the

Company to satisfy the exercise, strike or purchase price of a Stock Award granted under the Plan or a stock award granted under the Prior Plans (including any shares subject to such award that are not delivered because such award is exercised through a reduction of shares subject to such award (*i.e.*, "net exercised")); (B) any shares that are reacquired or withheld (or not issued) by the Company to satisfy a tax withholding obligation in connection with a Stock Award granted under the Plan or a stock award granted under the Prior Plans; (C) any shares repurchased by the Company on the open market with the proceeds of the exercise, strike or purchase price of a Stock Award granted under the Plan or a stock award granted under the Prior Plans; and (D) in the event that a Stock Appreciation Right granted under the Plan or a stock appreciation right granted under the Prior Plans is settled in shares of Common Stock, the gross number of shares of Common Stock subject to such award.

(c) Incentive Stock Option Limit. Subject to the provisions of Section 9(a) relating to Capitalization Adjustments, the aggregate maximum number of shares of Common Stock that may be issued pursuant to the exercise of Incentive Stock Options will be equal to 41,760,030.

(d) Section 162(m) Limitations. Subject to the provisions of Section 9(a) relating to Capitalization Adjustments, at such time as the Company may be subject to the applicable provisions of Section 162(m) of the Code, the following limitations shall apply.

(i) A maximum of 1,000,000 shares of Common Stock subject to Options, SARs and Other Stock Awards whose value is determined by reference to an increase over an exercise or strike price of at least 100% of the Fair Market Value on the date the Stock Award is granted may be granted to any one Participant during any one calendar year.

(ii) A maximum of 1,000,000 shares of Common Stock subject to Performance Stock Awards may be granted to any one Participant during any one calendar year (whether the grant, vesting or exercise is contingent upon the attainment during the Performance Period of the Performance Goals).

(iii) A maximum of $10,000,000 may be granted as a Performance Cash Award to any one Participant during any one calendar year.

(e) Limitation on Grants to Non-Employee Directors. The (i) maximum number of shares of Common Stock subject to Stock Awards granted under the Plan or otherwise during any one calendar year (beginning with the 2018 calendar year) to any Non-Employee Director, taken together with the (ii) cash fees paid by the Company to such Non-Employee Director during such calendar year, and in both cases for service on the Board, will not exceed $1,000,000 in total value (calculating the value of any such Stock Awards based on the grant date fair value of such Stock Awards for financial reporting purposes), or, with respect to the calendar year in which a Non-Employee Director is first appointed or elected to the Board, $1,500,000.

(f) Source of Shares. The stock issuable under the Plan will be shares of authorized but unissued or reacquired Common Stock, including shares repurchased by the Company on the open market or otherwise.

4. Eligibility.

(a) Eligibility for Specific Stock Awards. Incentive Stock Options may be granted only to employees of the Company or a "parent corporation" or "subsidiary corporation" thereof (as such terms are defined in Sections 424(e) and 424(f) of the Code). Stock Awards other than Incentive Stock Options may be granted to Employees, Directors and Consultants; *provided, however*, that Stock Awards may not be granted to Employees, Directors and Consultants who are providing Continuous Service only to any "parent" of the Company, as such term is defined in Rule 405 of the Securities Act, unless (i) the stock underlying such Stock Awards is treated as "service recipient stock" under Section 409A of the Code (for example, because the Stock Awards are granted pursuant to a corporate transaction such as a spin off transaction), (ii) the Company, in consultation with its legal counsel, has determined that such Stock Awards are otherwise exempt from Section 409A of the Code, or (iii) the Company, in consultation with its legal counsel, has determined that such Stock Awards comply with the distribution requirements of Section 409A of the Code.

(b) Ten Percent Shareholders. A Ten Percent Shareholder will not be granted an Incentive Stock Option unless the exercise price of such Option is at least 110% of the Fair Market Value on the date of grant and the Option is not exercisable after the expiration of five years from the date of grant.

5. Provisions Relating to Options and Stock Appreciation Rights.

Each Option or SAR will be in such form and will contain such terms and conditions as the Board deems appropriate. All Options will be separately designated Incentive Stock Options or Nonstatutory Stock Options at the time of grant, and, if certificates are issued, a separate certificate or certificates will be issued for shares of Common Stock purchased on exercise of each type of Option. If an Option is not specifically designated as an Incentive Stock Option, or if an Option is designated as an Incentive Stock Option but some portion or all of the Option fails to qualify as an Incentive Stock Option under the applicable rules, then the Option (or portion thereof) will be a Nonstatutory Stock Option. The provisions of separate Options or SARs need not be identical; *provided, however*, that each Award Agreement will conform to (through incorporation of provisions hereof by reference in the applicable Award Agreement or otherwise) the substance of each of the following provisions:

(a) Term. Subject to the provisions of Section 4(b) regarding Ten Percent Shareholders, no Option or SAR will be exercisable after the expiration of ten years from the date of its grant or such shorter period specified in the Award Agreement.

(b) Exercise Price. Subject to the provisions of Section 4(b) regarding Ten Percent Shareholders and except in the case of Substitute Awards, the exercise or strike price of each Option or SAR will be not less than 100% of the Fair Market Value of the Common Stock subject to the Option or SAR on the date the Award is granted. Notwithstanding the foregoing, an Option or SAR may be granted with an exercise or strike price lower than 100% of the Fair Market Value of the Common Stock subject to the Award if such Award is granted pursuant to an assumption of or substitution for another option or stock appreciation right pursuant to a Corporate Transaction and in a manner consistent with the provisions of Section 409A of the Code and, if applicable, Section 424(a) of the Code. Each SAR will be denominated in shares of Common Stock equivalents.

(c) Purchase Price for Options. The purchase price of Common Stock acquired pursuant to the exercise of an Option may be paid, to the extent permitted by applicable law and as determined by the Board in its sole discretion, by any combination of the methods of payment set forth below. The Board will have the authority to grant Options that do not permit all of the following methods of payment (or otherwise restrict the ability to use certain methods) and to grant Options that require the consent of the Company to use a particular method of payment. The permitted methods of payment are as follows:

(i) by cash, check, bank draft or money order payable to the Company;

(ii) pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of the stock subject to the Option, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds;

(iii) by delivery to the Company (either by actual delivery or attestation) of shares of Common Stock;

(iv) if an Option is a Nonstatutory Stock Option, by a “net exercise” arrangement pursuant to which the Company will reduce the number of shares of Common Stock issuable upon exercise by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price; *provided, however*, that the Company will accept a cash or other payment from the Participant to the extent of any remaining balance of the aggregate exercise price not satisfied by such reduction in the number of whole shares to be issued. Shares of Common Stock will no longer be subject to an Option and will not be exercisable thereafter to the extent that (A) shares issuable upon exercise are used to pay the exercise price pursuant to the “net exercise,” (B) shares are delivered to the Participant as a result of such exercise, and (C) shares are withheld to satisfy tax withholding obligations; or

(v) in any other form of legal consideration that may be acceptable to the Board and specified in the applicable Award Agreement.

(d) Exercise and Payment of a SAR. To exercise any outstanding SAR, the Participant must provide written notice of exercise to the Company in compliance with the provisions of the Stock Appreciation Right Agreement evidencing such SAR. The appreciation distribution payable on the exercise of a SAR will be not greater than an amount equal to the excess of (A) the aggregate Fair Market Value (on the date of the exercise of the SAR) of a number of shares of Common Stock equal to the number of Common Stock equivalents in which the Participant is vested under such SAR, and with respect to which the Participant is exercising the SAR on such date, over (B) the aggregate strike price of the number of Common Stock equivalents with respect to which the Participant is exercising the SAR on such date. The appreciation distribution may be paid in Common Stock, in cash, in any combination of

the two or in any other form of consideration, as determined by the Board and contained in the Award Agreement evidencing such SAR.

(e) Transferability of Options and SARs. The Board may, in its sole discretion, impose such limitations on the transferability of Options and SARs as the Board will determine. In the absence of such a determination by the Board to the contrary, the following restrictions on the transferability of Options and SARs will apply:

(i) Restrictions on Transfer. An Option or SAR will not be transferable except by will or by the laws of descent and distribution (or pursuant to subsections (ii) and (iii) below), and will be exercisable during the lifetime of the Participant only by the Participant. The Board may permit transfer of the Option or SAR in a manner that is not prohibited by applicable tax and securities laws. Except as explicitly provided in the Plan, neither an Option nor a SAR may be transferred for consideration.

(ii) Domestic Relations Orders. Subject to the approval of the Board or a duly authorized Officer, an Option or SAR may be transferred pursuant to the terms of a domestic relations order, official marital settlement agreement or other divorce or separation instrument as permitted by Treasury Regulations Section 1.421-1(b)(2). If an Option is an Incentive Stock Option, such Option may be deemed to be a Nonstatutory Stock Option as a result of such transfer.

(iii) Beneficiary Designation. Subject to the approval of the Board or a duly authorized Officer, a Participant may, by delivering written notice to the Company, in a form approved by the Company (or the designated broker), designate a third party who, on the death of the Participant, will thereafter be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, upon the death of the Participant, the executor or administrator of the Participant's estate will be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. However, the Company may prohibit designation of a beneficiary at any time, including due to any conclusion by the Company that such designation would be inconsistent with the provisions of applicable laws.

(f) Vesting Generally. The total number of shares of Common Stock subject to an Option or SAR may vest and become exercisable in periodic installments that may or may not be equal. The Option or SAR may be subject to such other terms and conditions on the time or times when it may or may not be exercised (which may be based on the satisfaction of Performance Goals or other criteria) as the Board may deem appropriate. The vesting provisions of individual Options or SARs may vary. The provisions of this Section 5(f) are subject to any Option or SAR provisions governing the minimum number of shares of Common Stock as to which an Option or SAR may be exercised.

(g) Termination of Continuous Service. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates (other than for Cause and other than upon the Participant's death or Disability), the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Award as of the date of termination of Continuous Service) within the period of time ending on the earlier of (i) the date three months following the termination of the Participant's Continuous Service (or such longer or shorter period specified in the applicable Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR (as applicable) within the applicable time frame, the Option or SAR will terminate.

(h) Extension of Termination Date. If the exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause and other than upon the Participant's death or Disability) would be prohibited at any time solely because the issuance of shares of Common Stock would violate the registration requirements under the Securities Act, then the Option or SAR will terminate on the earlier of (i) the expiration of a total period of time (that need not be consecutive) equal to the applicable post termination exercise period after the termination of the Participant's Continuous Service during which the exercise of the Option or SAR would not be in violation of such registration requirements, and (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement. In addition, unless otherwise provided in a Participant's Award Agreement, if the sale of any Common Stock received on exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause) would violate the Company's insider trading policy, then the Option or SAR will terminate on the earlier of (i) the expiration of a period of months (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the sale of the Common Stock received upon exercise of the Option or SAR would

not be in violation of the Company's insider trading policy, or (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement.

(i) Disability of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates as a result of the Participant's Disability, the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Option or SAR as of the date of termination of Continuous Service), but only within such period of time ending on the earlier of (i) the date 12 months following such termination of Continuous Service (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR within the applicable time frame, the Option or SAR (as applicable) will terminate.

(j) Death of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if (i) a Participant's Continuous Service terminates as a result of the Participant's death, or (ii) the Participant dies within the period (if any) specified in the Award Agreement for exercisability after the termination of the Participant's Continuous Service for a reason other than death, then the Option or SAR may be exercised (to the extent the Participant was entitled to exercise such Option or SAR as of the date of death) by the Participant's estate, by a person who acquired the right to exercise the Option or SAR by bequest or inheritance or by a person designated to exercise the Option or SAR upon the Participant's death, but only within the period ending on the earlier of (i) the date 12 months following the date of death (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of such Option or SAR as set forth in the Award Agreement. If, after the Participant's death, the Option or SAR is not exercised within the applicable time frame, the Option or SAR (as applicable) will terminate.

(k) Termination for Cause. Except as explicitly provided otherwise in a Participant's Award Agreement or other individual written agreement between the Company or any Affiliate and the Participant, if a Participant's Continuous Service is terminated for Cause, the Option or SAR will terminate immediately upon such Participant's termination of Continuous Service, and the Participant will be prohibited from exercising his or her Option or SAR from and after the time of such termination of Continuous Service.

(l) Non-Exempt Employees. If an Option or SAR is granted to an Employee who is a non-exempt employee for purposes of the Fair Labor Standards Act of 1938, as amended, the Option or SAR will not be first exercisable for any shares of Common Stock until at least six months following the date of grant of the Option or SAR (although the Award may vest prior to such date). Consistent with the provisions of the Worker Economic Opportunity Act, (i) if such non-exempt Employee dies or suffers a Disability, (ii) upon a Corporate Transaction in which such Option or SAR is not assumed, continued, or substituted, (iii) upon a Change in Control, or (iv) upon the Participant's retirement (as such term may be defined in the Participant's Award Agreement in another agreement between the Participant and the Company, or, if no such definition, in accordance with the Company's then current employment policies and guidelines), the vested portion of any Options and SARs may be exercised earlier than six months following the date of grant. The foregoing provision is intended to operate so that any income derived by a non-exempt employee in connection with the exercise or vesting of an Option or SAR will be exempt from his or her regular rate of pay. To the extent permitted and/or required for compliance with the Worker Economic Opportunity Act to ensure that any income derived by a non-exempt employee in connection with the exercise, vesting or issuance of any shares under any other Stock Award will be exempt from the employee's regular rate of pay, the provisions of this Section 5(l) will apply to all Stock Awards and are hereby incorporated by reference into such Stock Award Agreements.

6. Provisions of Stock Awards other than Options and SARs.

(a) Restricted Stock Awards. Each Restricted Stock Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. To the extent consistent with the Company's bylaws, at the Board's election, shares of Common Stock may be (x) held in book entry form subject to the Company's instructions until any restrictions relating to the Restricted Stock Award lapse; or (y) evidenced by a certificate, which certificate will be held in such form and manner as determined by the Board. The terms and conditions of Restricted Stock Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Award Agreements need not be identical. Each Restricted Stock Award Agreement will conform to (through incorporation of the provisions hereof by reference in the agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. A Restricted Stock Award may be awarded in consideration for (A) cash, check, bank draft or money order payable to the Company, (B) past services to the Company or an Affiliate, or (C) any other form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. Shares of Common Stock awarded under the Restricted Stock Award Agreement may be subject to forfeiture to the Company in accordance with a vesting schedule to be determined by the Board.

(iii) Termination of Participant's Continuous Service. If a Participant's Continuous Service terminates, the Company may receive through a forfeiture condition or a repurchase right any or all of the shares of Common Stock held by the Participant that have not vested as of the date of termination of Continuous Service under the terms of the Restricted Stock Award Agreement.

(iv) Transferability. Rights to acquire shares of Common Stock under the Restricted Stock Award Agreement will be transferable by the Participant only upon such terms and conditions as are set forth in the Restricted Stock Award Agreement, as the Board will determine in its sole discretion, so long as Common Stock awarded under the Restricted Stock Award Agreement remains subject to the terms of the Restricted Stock Award Agreement.

(b) Restricted Stock Unit Awards. Each Restricted Stock Unit Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. The terms and conditions of Restricted Stock Unit Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Unit Award Agreements need not be identical. Each Restricted Stock Unit Award Agreement will conform to (through incorporation of the provisions hereof by reference in the Agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. At the time of grant of a Restricted Stock Unit Award, the Board will determine the consideration, if any, to be paid by the Participant upon delivery of each share of Common Stock subject to the Restricted Stock Unit Award. The consideration to be paid (if any) by the Participant for each share of Common Stock subject to a Restricted Stock Unit Award may be paid in any form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. At the time of the grant of a Restricted Stock Unit Award, the Board may impose such restrictions on or conditions to the vesting of the Restricted Stock Unit Award as it, in its sole discretion, deems appropriate.

(iii) Payment. A Restricted Stock Unit Award may be settled by the delivery of shares of Common Stock, their cash equivalent, any combination thereof or in any other form of consideration, as determined by the Board and contained in the Restricted Stock Unit Award Agreement.

(iv) Additional Restrictions. At the time of the grant of a Restricted Stock Unit Award, the Board, as it deems appropriate, may impose such restrictions or conditions that delay the delivery of the shares of Common Stock (or their cash equivalent) subject to a Restricted Stock Unit Award to a time after the vesting of such Restricted Stock Unit Award.

(v) Termination of Participant's Continuous Service. Except as otherwise provided in the applicable Restricted Stock Unit Award Agreement, such portion of the Restricted Stock Unit Award that has not vested will be forfeited upon the Participant's termination of Continuous Service.

(c) Performance Awards.

(i) Performance Stock Awards. A Performance Stock Award is a Stock Award (covering a number of shares not in excess of that set forth in Section 3(d) above) that is payable (including that may be granted, may vest or may be exercised) contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Stock Award may, but need not, require the Participant's completion of a specified period of Continuous Service. The length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Committee (or, to the extent that an Award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Board or the Committee), in its sole discretion. In addition, to the extent permitted by applicable law and the applicable Award Agreement, the Board or the Committee may determine that cash may be used in payment of Performance Stock Awards.

(ii) Performance Cash Awards. A Performance Cash Award is a cash award (for a dollar value not in excess of that set forth in Section 3(d) above) that is payable contingent upon the attainment during a Performance Period of

certain Performance Goals. A Performance Cash Award may also require the completion of a specified period of Continuous Service. At the time of grant of a Performance Cash Award, the length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Committee (or, to the extent that an Award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Board or the Committee), in its sole discretion. The Board or the Committee may specify the form of payment of Performance Cash Awards, which may be cash or other property, or may provide for a Participant to have the option for his or her Performance Cash Award, or such portion thereof as the Board or the Committee may specify, to be paid in whole or in part in cash or other property.

(iii) Committee and Board Discretion. With respect to any Performance Stock Award or Performance Cash Award, the Committee (or, to the extent that an Award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Board or the Committee) retains the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of Performance Goals and to define the manner of calculating the Performance Criteria it selects to use for a Performance Period. Partial achievement of the specified criteria may result in the payment or vesting corresponding to the degree of achievement as specified in the Stock Award Agreement or the written terms of a Performance Cash Award.

(iv) Section 162(m) Compliance. Unless otherwise permitted in compliance with the requirements of Section 162(m) of the Code with respect to an Award intended to qualify as "performance-based compensation" thereunder, the Committee will establish the Performance Goals applicable to, and the formula for calculating the amount payable under, the Award no later than the earlier of (a) the date 90 days after the commencement of the applicable Performance Period, and (b) the date on which 25% of the Performance Period has elapsed, and in any event at a time when the achievement of the applicable Performance Goals remains substantially uncertain. Prior to the payment of any compensation under an Award intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Committee will certify the extent to which any Performance Goals and any other material terms under such Award have been satisfied (other than in cases where such Performance Goals relate solely to the increase in the value of the Common Stock). Notwithstanding satisfaction of, or completion of any Performance Goals, the number of shares of Common Stock, Options, cash or other benefits granted, issued, retainable and/or vested under an Award on account of satisfaction of such Performance Goals may be reduced by the Committee on the basis of such further considerations as the Committee, in its sole discretion, will determine.

(d) Other Stock Awards. Other forms of Stock Awards valued in whole or in part by reference to, or otherwise based on, Common Stock, including the appreciation in value thereof (e.g., options or stock rights with an exercise price or strike price less than 100% of the Fair Market Value of the Common Stock at the time of grant) may be granted either alone or in addition to Stock Awards provided for under Section 5 and the preceding provisions of this Section 6. Subject to the provisions of the Plan, the Board will have sole and complete authority to determine the persons to whom and the time or times at which such Other Stock Awards will be granted, the number of shares of Common Stock (or the cash equivalent thereof) to be granted pursuant to such Other Stock Awards and all other terms and conditions of such Other Stock Awards.

7. Covenants of the Company.

(a) Availability of Shares. The Company will keep available at all times the number of shares of Common Stock reasonably required to satisfy then-outstanding Awards.

(b) Securities Law Compliance. The Company will seek to obtain from each regulatory commission or agency having jurisdiction over the Plan such authority as may be required to grant Stock Awards and to issue and sell shares of Common Stock upon exercise of the Stock Awards; *provided, however,* that this undertaking will not require the Company to register under the Securities Act the Plan, any Stock Award or any Common Stock issued or issuable pursuant to any such Stock Award. If, after reasonable efforts and at a reasonable cost, the Company is unable to obtain from any such regulatory commission or agency the authority that counsel for the Company deems necessary for the lawful issuance and sale of Common Stock under the Plan, the Company will be relieved from any liability for failure to issue and sell Common Stock upon exercise of such Stock Awards unless and until such authority is obtained. A Participant will not be eligible for the grant of an Award or the subsequent issuance of cash or Common Stock pursuant to the Award if such grant or issuance would be in violation of any applicable securities law.

(c) No Obligation to Notify or Minimize Taxes. The Company will have no duty or obligation to any Participant to advise such holder as to the time or manner of exercising such Stock Award. Furthermore, the Company will have

no duty or obligation to warn or otherwise advise such holder of a pending termination or expiration of an Award or a possible period in which the Award may not be exercised. The Company has no duty or obligation to minimize the tax consequences of an Award to the holder of such Award.

8. Miscellaneous.

(a) Use of Proceeds from Sales of Common Stock. Proceeds from the sale of shares of Common Stock pursuant to Awards will constitute general funds of the Company.

(b) Corporate Action Constituting Grant of Awards. Corporate action constituting a grant by the Company of an Award to any Participant will be deemed completed as of the date of such corporate action, unless otherwise determined by the Board, regardless of when the instrument, certificate, or letter evidencing the Award is communicated to, or actually received or accepted by, the Participant. In the event that the corporate records (e.g., Board consents, resolutions or minutes) documenting the corporate action constituting the grant contain terms (e.g., exercise price, vesting schedule or number of shares) that are inconsistent with those in the Award Agreement or related grant documents as a result of a clerical error in the papering of the Award Agreement or related grant documents, the corporate records will control and the Participant will have no legally binding right to the incorrect term in the Award Agreement or related grant documents.

(c) Shareholder Rights. No Participant will be deemed to be the holder of, or to have any of the rights of a holder with respect to, any shares of Common Stock subject to an Award unless and until (i) such Participant has satisfied all requirements for exercise of, or the issuance of shares of Common Stock under, the Award pursuant to its terms, and (ii) the issuance of the Common Stock subject to such Award has been entered into the books and records of the Company.

(d) No Employment or Other Service Rights. Nothing in the Plan, any Award Agreement or any other instrument executed thereunder or in connection with any Award granted pursuant thereto will confer upon any Participant any right to continue to serve the Company or an Affiliate in the capacity in effect at the time the Award was granted or will affect the right of the Company or an Affiliate to terminate (i) the employment of an Employee with or without notice and with or without cause, (ii) the service of a Consultant pursuant to the terms of such Consultant's agreement with the Company or an Affiliate, or (iii) the service of a Director pursuant to the bylaws of the Company or an Affiliate, and any applicable provisions of the corporate law of the state in which the Company or the Affiliate is incorporated, as the case may be.

(e) Change in Time Commitment. In the event a Participant's regular level of time commitment in the performance of his or her services for the Company and any Affiliates is reduced (for example, and without limitation, if the Participant is an Employee of the Company and the Employee has a change in status from a full-time Employee to a part-time Employee or takes an extended leave of absence) after the date of grant of any Award to the Participant, the Board has the right in its sole discretion to (x) make a corresponding reduction in the number of shares or cash amount subject to any portion of such Award that is scheduled to vest or become payable after the date of such change in time commitment, and (y) in lieu of or in combination with such a reduction, extend the vesting or payment schedule applicable to such Award. In the event of any such reduction, the Participant will have no right with respect to any portion of the Award that is so reduced or extended.

(f) Incentive Stock Option Limitations. To the extent that the aggregate Fair Market Value (determined at the time of grant) of Common Stock with respect to which Incentive Stock Options are exercisable for the first time by any Optionholder during any calendar year (under all plans of the Company and any Affiliates) exceeds $100,000 (or such other limit established in the Code) or otherwise does not comply with the rules governing Incentive Stock Options, the Options or portions thereof that exceed such limit (according to the order in which they were granted) or otherwise do not comply with such rules will be treated as Nonstatutory Stock Options, notwithstanding any contrary provision of the applicable Option Agreement(s).

(g) Investment Assurances. The Company may require a Participant, as a condition of exercising or acquiring Common Stock under any Award, (i) to give written assurances satisfactory to the Company as to the Participant's knowledge and experience in financial and business matters and/or to employ a purchaser representative reasonably satisfactory to the Company who is knowledgeable and experienced in financial and business matters and that such Participant is capable of evaluating, alone or together with the purchaser representative, the merits and risks of exercising the Award; and (ii) to give written assurances satisfactory to the Company stating that the Participant is acquiring Common Stock subject to the Award for the Participant's own account and not with any present intention of

selling or otherwise distributing the Common Stock. The foregoing requirements, and any assurances given pursuant to such requirements, will be inoperative if (A) the issuance of the shares upon the exercise or acquisition of Common Stock under the Award has been registered under a then currently effective registration statement under the Securities Act, or (B) as to any particular requirement, a determination is made by counsel for the Company that such requirement need not be met in the circumstances under the then applicable securities laws. The Company may, upon advice of counsel to the Company, place legends on stock certificates issued under the Plan as such counsel deems necessary or appropriate in order to comply with applicable securities laws, including, but not limited to, legends restricting the transfer of the Common Stock.

(h) Withholding Obligations. Unless prohibited by the terms of an Award Agreement, the Company may, in its sole discretion, satisfy any federal, state or local tax withholding obligation relating to an Award by any of the following means or by a combination of such means: (i) causing the Participant to tender a cash payment; (ii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to the Participant in connection with the Award; *provided, however,* that no shares of Common Stock are withheld with a value exceeding an amount of tax calculated based on the maximum statutory tax rates in a Participant's applicable tax jurisdiction (or such other amount as may be necessary to avoid classification of the Stock Award as a liability for financial accounting purposes); (iii) withholding cash from an Award settled in cash; (iv) withholding payment from any amounts otherwise payable to the Participant; or (v) by such other method as may be set forth in the Award Agreement.

(i) Electronic Delivery. Any reference herein to a "written" agreement or document will include any agreement or document delivered electronically, filed publicly at *www.sec.gov* (or any successor website thereto) or posted on the Company's intranet (or other shared electronic medium controlled by the Company to which the Participant has access).

(j) Deferrals. To the extent permitted by applicable law, the Board, in its sole discretion, may determine that the delivery of Common Stock or the payment of cash, upon the exercise, vesting or settlement of all or a portion of any Award may be deferred and may establish programs and procedures for deferral elections to be made by Participants. Deferrals by Participants will be made in accordance with Section 409A of the Code. Consistent with Section 409A of the Code, the Board may provide for distributions while a Participant is still an employee or otherwise providing services to the Company. The Board is authorized to make deferrals of Awards and determine when, and in what annual percentages, Participants may receive payments, including lump sum payments, following the Participant's termination of Continuous Service, and implement such other terms and conditions consistent with the provisions of the Plan and in accordance with applicable law.

(k) Compliance with Section 409A of the Code. Unless otherwise expressly provided for in an Award Agreement, the Plan and Award Agreements will be interpreted to the greatest extent possible in a manner that makes the Plan and the Awards granted hereunder exempt from Section 409A of the Code, and, to the extent not so exempt, in compliance with Section 409A of the Code. If the Board determines that any Award granted hereunder is not exempt from and is therefore subject to Section 409A of the Code, the Award Agreement evidencing such Award will incorporate the terms and conditions necessary to avoid the consequences specified in Section 409A(a)(1) of the Code, and to the extent an Award Agreement is silent on terms necessary for compliance, such terms are hereby incorporated by reference into the Award Agreement. Notwithstanding anything to the contrary in this Plan (and unless the Award Agreement specifically provides otherwise), if the shares of Common Stock are publicly traded, and if a Participant holding an Award that constitutes "deferred compensation" under Section 409A of the Code is a "specified employee" for purposes of Section 409A of the Code, no distribution or payment of any amount that is due because of a "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) will be issued or paid before the date that is six months following the date of such Participant's "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) or, if earlier, the date of the Participant's death, unless such distribution or payment can be made in a manner that complies with Section 409A of the Code, and any amounts so deferred will be paid in a lump sum on the day after such six month period elapses, with the balance paid thereafter on the original schedule.

(l) Clawback/Recovery. All Awards granted under the Plan will be subject to recoupment in accordance with any clawback policy that the Company is required to adopt pursuant to the listing standards of any national securities exchange or association on which the Company's securities are listed or as is otherwise required by the Dodd-Frank Wall Street Reform and Consumer Protection Act or other applicable law. In addition, the Board may impose such other clawback, recovery or recoupment provisions in an Award Agreement as the Board determines necessary or

appropriate, including but not limited to a reacquisition right in respect of previously acquired shares of Common Stock or other cash or property upon the occurrence of an event constituting Cause. No recovery of compensation under such a clawback policy will be an event giving rise to a right to resign for "good reason" or "constructive termination" (or similar term) under any agreement with the Company.

(m) Dividends and Dividend Equivalents. Dividends and dividend equivalents may be credited in respect of shares of Common Stock covered by a Stock Award (other than Options and Stock Appreciation Rights), as determined by the Board and contained in the applicable Award Agreement. At the sole discretion of the Board, such dividends and dividend equivalents may be converted into additional shares of Common Stock covered by the Stock Award in such manner as determined by the Board.

Any additional shares or cash payments covered by the Stock Award credited by reason of such dividends or dividend equivalents will be subject to all of the same terms and conditions of the underlying Award Agreement to which they relate. Notwithstanding anything to the contrary in this Plan or any Award Agreement, dividends and dividend equivalents shall not be paid in respect of shares of Common Stock covered by a Stock Award until such shares of Common Stock vest pursuant to the applicable Award Agreement.

9. Adjustments upon Changes in Common Stock; Other Corporate Events.

(a) Capitalization Adjustments. In the event of a Capitalization Adjustment, the Board will appropriately and proportionately adjust: (i) the class(es) and maximum number of securities subject to the Plan pursuant to Section 3(a), (ii) the class(es) and maximum number of securities that may be issued pursuant to the exercise of Incentive Stock Options pursuant to Section 3(c), (iii) the class(es) and maximum number of securities that may be awarded to any person pursuant to Sections 3(d), and (iv) the class(es) and number of securities and price per share of stock subject to outstanding Stock Awards. The Board will make such adjustments, and its determination will be final, binding and conclusive.

(b) Dissolution. Except as otherwise provided in the Stock Award Agreement, in the event of a Dissolution of the Company, all outstanding Stock Awards (other than Stock Awards consisting of vested and outstanding shares of Common Stock not subject to a forfeiture condition or the Company's right of repurchase) will terminate immediately prior to the completion of such Dissolution, and the shares of Common Stock subject to the Company's repurchase rights or subject to a forfeiture condition may be repurchased or reacquired by the Company notwithstanding the fact that the holder of such Stock Award is providing Continuous Service; *provided, however*, that the Board may, in its sole discretion, cause some or all Stock Awards to become fully vested, exercisable and/or no longer subject to repurchase or forfeiture (to the extent such Stock Awards have not previously expired or terminated) before the Dissolution is completed but contingent on its completion.

(c) Transactions. The following provisions shall apply to Stock Awards in the event of a Transaction unless otherwise provided in the instrument evidencing the Stock Award or any other written agreement between the Company or any Affiliate and the Participant or unless otherwise expressly provided by the Board at the time of grant of a Stock Award. In the event of a Transaction, then, notwithstanding any other provision of the Plan, the Board shall take one or more of the following actions with respect to Stock Awards, contingent upon the closing or completion of the Transaction:

(i) arrange for the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company) to assume or continue the Stock Award or to substitute a similar stock award for the Stock Award (including, but not limited to, an award to acquire the same consideration paid to the shareholders of the Company pursuant to the Transaction);

(ii) arrange for the assignment of any reacquisition or repurchase rights held by the Company in respect of Common Stock issued pursuant to the Stock Award to the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company);

(iii) accelerate the vesting, in whole or in part, of the Stock Award (and, if applicable, the time at which the Stock Award may be exercised) to a date prior to the effective time of such Transaction as the Board shall determine (or, if the Board shall not determine such a date, to the date that is five days prior to the effective date of the Transaction), with such Stock Award terminating if not exercised (if applicable) at or prior to the effective time of the Transaction;

(iv) arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by the Company with respect to the Stock Award;

(v) cancel or arrange for the cancellation of the Stock Award, to the extent not vested or not exercised prior to the effective time of the Transaction, in exchange for such cash consideration, if any, as the Board, in its sole discretion, may consider appropriate; and

(vi) make a payment, in such form as may be determined by the Board equal to the excess, if any, of (A) the value of the property the Participant would have received upon the exercise of the Stock Award immediately prior to the effective time of the Transaction, over (B) any exercise price payable by such holder in connection with such exercise. For clarity, this payment may be zero ($0) if the value of the property is equal to or less than the exercise price. Payments under this provision may be delayed to the same extent that payment of consideration to the holders of Common Stock in connection with the Transaction is delayed as a result of escrows, earn outs, holdbacks or other contingencies.

The Board need not take the same action or actions with respect to all Stock Awards or portions thereof or with respect to all Participants. The Board may take different actions with respect to the vested and unvested portions of a Stock Award.

(d) Change in Control. A Stock Award may be subject to additional acceleration of vesting and exercisability upon or after a Change in Control as may be provided in the Stock Award Agreement for such Stock Award or as may be provided in any other written agreement between the Company or any Affiliate and the Participant, but in the absence of such provision, no such acceleration will occur.

10. Plan Term; Earlier Termination or Suspension of the Plan.

The Board may suspend or terminate the Plan at any time. No Incentive Stock Options may be granted after the tenth anniversary of the earlier of (i) the date the Plan is adopted by the Board (the "***Adoption Date***"), or (ii) the date the Plan is approved by the shareholders of the Company. No Awards may be granted under the Plan while the Plan is suspended or after it is terminated. Suspension or termination of the Plan will not impair rights and obligations under any Award granted while the Plan is in effect except with the written consent of the affected Participant or as otherwise permitted in the Plan.

11. Existence of the Plan.

The Plan will become effective on the Effective Date.

12. Choice of Law.

The law of the State of Delaware will govern all questions concerning the construction, validity and interpretation of this Plan, without regard to that state's conflict of laws rules.

13. Definitions. As used in the Plan, the following definitions will apply to the capitalized terms indicated below:

(a) "***Affiliate***" means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405 of the Securities Act. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

(b) "***Award***" means a Stock Award or a Performance Cash Award.

(c) "***Award Agreement***" means a written agreement between the Company and a Participant evidencing the terms and conditions of an Award.

(d) "***Board***" means the Board of Directors of the Company.

(e) "***Capital Stock***" means each and every class of common stock of the Company, regardless of the number of votes per share.

(f) "***Capitalization Adjustment***" means any change that is made in, or other events that occur with respect to, the Common Stock subject to the Plan or subject to any Stock Award after the Adoption Date without the receipt of consideration by the Company through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, reverse stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or any similar equity

restructuring transaction, as that term is used in Statement of Financial Accounting Standards Board Accounting Standards Codification Topic 718 (or any successor thereto). Notwithstanding the foregoing, the conversion of any convertible securities of the Company will not be treated as a Capitalization Adjustment.

(g) *"**Cause**"* shall have the meaning ascribed to such term in any written agreement between the Participant and the Company defining such term and, in the absence of such agreement, such term means, with respect to a Participant, the occurrence of any of the following events: (i) such Participant's commission of any felony or any crime involving fraud, dishonesty or moral turpitude under the laws of the United States or any state thereof; (ii) such Participant's attempted commission of, or participation in, a fraud or act of dishonesty against the Company; (iii) such Participant's intentional, material violation of any contract or agreement between the Participant and the Company or of any statutory duty owed to the Company; (iv) such Participant's unauthorized use or disclosure of the Company's confidential information or trade secrets; or (v) such Participant's gross misconduct. The determination that a termination of the Participant's Continuous Service is either for Cause or without Cause shall be made by the Company, in its sole discretion. Any determination by the Company that the Continuous Service of a Participant was terminated with or without Cause for the purposes of outstanding Awards held by such Participant shall have no effect upon any determination of the rights or obligations of the Company or such Participant for any other purpose.

(h) *"**Change in Control**"* means the occurrence, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) any Exchange Act Person becomes the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company's then outstanding securities other than by virtue of a merger, consolidation or similar transaction. Notwithstanding the foregoing, a Change in Control will not be deemed to occur (A) on account of the acquisition of securities of the Company directly from the Company; (B) on account of the acquisition of securities of the Company by an investor, any affiliate thereof or any other Exchange Act Person that acquires the Company's securities in a transaction or series of related transactions the primary purpose of which is to obtain financing for the Company through the issuance of equity securities; or (C) solely because the level of Ownership held by any Exchange Act Person (the "***Subject Person***") exceeds the designated percentage threshold of the outstanding voting securities as a result of a repurchase or other acquisition of voting securities by the Company reducing the number of shares outstanding, provided that if a Change in Control would occur (but for the operation of this sentence) as a result of the acquisition of voting securities by the Company, and after such share acquisition, the Subject Person becomes the Owner of any additional voting securities that, assuming the repurchase or other acquisition had not occurred, increases the percentage of the then outstanding voting securities Owned by the Subject Person over the designated percentage threshold, then a Change in Control will be deemed to occur;

(ii) there is consummated a merger, consolidation or similar transaction involving (directly or indirectly) the Company and, immediately after the consummation of such merger, consolidation or similar transaction, the shareholders of the Company immediately prior thereto do not Own, directly or indirectly, either (A) outstanding voting securities representing more than 50% of the combined outstanding voting power of the surviving Entity in such merger, consolidation or similar transaction or (B) more than 50% of the combined outstanding voting power of the parent of the surviving Entity in such merger, consolidation or similar transaction, in each case in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such transaction;

(iii) there is consummated a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries, other than a sale, lease, license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries to an Entity, more than 50% of the combined voting power of the voting securities of which are Owned by shareholders of the Company in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such sale, lease, license or other disposition;

(iv) the complete dissolution or liquidation of the Company, except for a liquidation into a parent corporation;

(v) individuals who, on the date the Plan is adopted by the Board, are members of the Board (the "***Incumbent Board***") cease for any reason to constitute at least a majority of the members of the Board; *provided, however*, that if the appointment or election (or nomination for election) of any new Board member was approved or recommended by a majority vote of the members of the Incumbent Board then still in office, such new member will, for purposes of this Plan, be considered as a member of the Incumbent Board.

Notwithstanding the foregoing definition or any other provision of the Plan, the term Change in Control will not include a sale of assets, merger or other transaction effected exclusively for the purpose of changing the domicile of the Company and the definition of Change in Control (or any analogous term) in an individual written agreement between the Company or any Affiliate and the Participant will supersede the foregoing definition with respect to Awards subject to such agreement; *provided, however*, that if no definition of Change in Control or any analogous term is set forth in such an individual written agreement, the foregoing definition will apply.

If required for compliance with Section 409A of the Code, in no event will an event be deemed a Change in Control if such event is not also a "change in the ownership of" the Company, a "change in the effective control of" the Company or a "change in the ownership of a substantial portion of the assets of" the Company, each as determined under Treasury Regulations Section 1.409A-3(i)(5) (without regard to any alternative definition thereunder).

(i) "***Code***" means the Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder.

(j) "***Committee***" means a committee of one or more Directors to whom authority has been delegated by the Board in accordance with Section 2(c).

(k) "***Common Stock****"* means the common stock of the Company, having one vote per share.

(l) "***Company***" means BioMarin Pharmaceutical Inc..

(m) "***Consultant***" means any person, including an advisor, who is (i) engaged by the Company or an Affiliate to render consulting or advisory services and is compensated for such services, or (ii) serving as a member of the board of directors of an Affiliate and is compensated for such services. However, service solely as a Director, or payment of a fee for such service, will not cause a Director to be considered a "Consultant" for purposes of the Plan. Notwithstanding the foregoing, a person is treated as a Consultant under this Plan only if a Form S-8 Registration Statement under the Securities Act is available to register either the offer or the sale of the Company's securities to such person.

(n) "***Continuous Service***" means that the Participant's service with the Company or an Affiliate, whether as an Employee, Director or Consultant, is not interrupted or terminated. A change in the capacity in which the Participant renders service to the Company or an Affiliate as an Employee, Consultant or Director or a change in the entity for which the Participant renders such service, provided that there is no interruption or termination of the Participant's service with the Company or an Affiliate, will not terminate a Participant's Continuous Service; *provided, however*, that if the Entity for which a Participant is rendering services ceases to qualify as an Affiliate, as determined by the Board, in its sole discretion, such Participant's Continuous Service will be considered to have terminated on the date such Entity ceases to qualify as an Affiliate. To the extent permitted by law, the Board or the chief executive officer of the Company, in that party's sole discretion, may determine whether Continuous Service will be considered interrupted in the case of (i) any leave of absence approved by the Board or chief executive officer, including sick leave, military leave or any other personal leave, or (ii) transfers between the Company, an Affiliate, or their successors. Notwithstanding the foregoing, a leave of absence will be treated as Continuous Service for purposes of vesting in an Award only to such extent as may be provided in the Company's leave of absence policy, in the written terms of any leave of absence agreement or policy applicable to the Participant, or as otherwise required by law.

(o) "***Corporate Transaction***" means the consummation, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) a sale or other disposition of all or substantially all, as determined by the Board, in its sole discretion, of the consolidated assets of the Company and its Subsidiaries;

(ii) a sale or other disposition of more than 50% of the outstanding securities of the Company;

(iii) a merger, consolidation or similar transaction following which the Company is not the surviving corporation; or

(iv) a merger, consolidation or similar transaction following which the Company is the surviving corporation but the shares of Common Stock outstanding immediately preceding the merger, consolidation or similar transaction are converted or exchanged by virtue of the merger, consolidation or similar transaction into other property, whether in the form of securities, cash or otherwise.

If required for compliance with Section 409A of the Code, in no event will an event be deemed a Corporate Transaction if such event is not also a "change in the ownership of" the Company, a "change in the effective control of" the Company or a "change in the ownership of a substantial portion of the assets of" the Company, each as determined under Treasury Regulations Section 1.409A-3(i)(5) (without regard to any alternative definition thereunder).

(p) "***Covered Employee***" will have the meaning provided in Section 162(m)(3) of the Code.

(q) "***Director*** " means a member of the Board.

(r) "***Disability***" means, with respect to a Participant, the inability of such Participant to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment that can be expected to result in death or that has lasted or can be expected to last for a continuous period of not less than 12 months, as provided in Sections 22(e)(3) and 409A(a) (2)(c)(i) of the Code, and will be determined by the Board on the basis of such medical evidence as the Board deems warranted under the circumstances.

(s) "***Dissolution***" means when the Company, after having executed a certificate of dissolution with the State of Delaware (or other applicable state), has completely wound up its affairs. Conversion of the Company into a Limited Liability Company (or any other pass-through entity) will not be considered a "Dissolution" for purposes of the Plan.

(t) "***Effective Date***" means the date of the Company shareholders approve this Plan, which is the date of the annual meeting of shareholders of the Company held on June 6, 2017, provided this Plan is approved by the Company's shareholders at such meeting.

(u) "***Employee***" means any person employed by the Company or an Affiliate. However, service solely as a Director, or payment of a fee for such services, will not cause a Director to be considered an "Employee" for purposes of the Plan.

(v) "***Entity***" means a corporation, partnership, limited liability company or other entity.

(w) "***Exchange Act***" means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

(x) "***Exchange Act Person***" means any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act), except that "Exchange Act Person" will not include (i) the Company or any Subsidiary of the Company, (ii) any employee benefit plan of the Company or any Subsidiary of the Company or any trustee or other fiduciary holding securities under an employee benefit plan of the Company or any Subsidiary of the Company, (iii) an underwriter temporarily holding securities pursuant to a registered public offering of such securities, (iv) an Entity Owned, directly or indirectly, by the shareholders of the Company in substantially the same proportions as their Ownership of stock of the Company; or (v) any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act) that, as of the Effective Date, is the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company's then outstanding securities.

(y) "***Fair Market Value***" means, as of any date, the value of the Common Stock determined as follows:

(i) If the Common Stock is listed on any established stock exchange or traded on any established market, the Fair Market Value of a share of Common Stock will be, unless otherwise determined by the Board, the closing sales price for such stock as quoted on such exchange or market (or the exchange or market with the greatest volume of trading in the Common Stock) on the date of determination, as reported in a source the Board deems reliable.

(ii) Unless otherwise provided by the Board, if there is no closing sales price for the Common Stock on the date of determination, then the Fair Market Value will be the closing selling price on the last preceding date for which such quotation exists.

(iii) In the absence of such markets for the Common Stock, the Fair Market Value will be determined by the Board in good faith and in a manner that complies with Sections 409A and 422 of the Code.

(z) "***Incentive Stock Option***" means an option granted pursuant to Section 5 of the Plan that is intended to be, and qualifies as, an "incentive stock option" within the meaning of Section 422 of the Code.

(aa) "***Non-Employee Director***" means a Director who either (i) is not a current employee or officer of the Company or an Affiliate, does not receive compensation, either directly or indirectly, from the Company or an Affiliate for services rendered as a consultant or in any capacity other than as a Director (except for an amount as to which

disclosure would not be required under Item 404(a) of Regulation S-K promulgated pursuant to the Securities Act ("***Regulation S-K***")), does not possess an interest in any other transaction for which disclosure would be required under Item 404(a) of Regulation S-K, and is not engaged in a business relationship for which disclosure would be required pursuant to Item 404(b) of Regulation S-K; or (ii) is otherwise considered a "non-employee director" for purposes of Rule 16b-3.

(bb) "***Nonstatutory Stock Option***" means any Option granted pursuant to Section 5 of the Plan that does not qualify as an Incentive Stock Option.

(cc) "***Officer***" means a person who is an officer of the Company within the meaning of Section 16 of the Exchange Act.

(dd) "***Option***" means an Incentive Stock Option or a Nonstatutory Stock Option to purchase shares of Common Stock granted pursuant to the Plan.

(ee) "***Option Agreement***" means a written agreement between the Company and an Optionholder evidencing the terms and conditions of an Option grant. Each Option Agreement will be subject to the terms and conditions of the Plan.

(ff) "***Optionholder***" means a person to whom an Option is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Option.

(gg) "***Other Stock Award***" means an award based in whole or in part by reference to the Common Stock which is granted pursuant to the terms and conditions of Section 6(d).

(hh) "***Other Stock Award Agreement***" means a written agreement between the Company and a holder of an Other Stock Award evidencing the terms and conditions of an Other Stock Award grant. Each Other Stock Award Agreement will be subject to the terms and conditions of the Plan.

(ii) "***Outside Director***" means a Director who either (i) is not a current employee of the Company or an "affiliated corporation" (within the meaning of Treasury Regulations promulgated under Section 162(m) of the Code), is not a former employee of the Company or an "affiliated corporation" who receives compensation for prior services (other than benefits under a tax-qualified retirement plan) during the taxable year, has not been an officer of the Company or an "affiliated corporation," and does not receive remuneration from the Company or an "affiliated corporation," either directly or indirectly, in any capacity other than as a Director, or (ii) is otherwise considered an "outside director" for purposes of Section 162(m) of the Code.

(jj) "***Own,***" "***Owned,***" "***Owner,***" "***Ownership***" means a person or Entity will be deemed to "Own," to have "Owned," to be the "Owner" of, or to have acquired "Ownership" of securities if such person or Entity, directly or indirectly, through any contract, arrangement, understanding, relationship or otherwise, has or shares voting power, which includes the power to vote or to direct the voting, with respect to such securities.

(kk) "***Participant***" means a person to whom an Award is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Stock Award.

(ll) "***Performance Cash Award***" means an award of cash granted pursuant to the terms and conditions of Section 6(c)(ii).

(mm) "***Performance Criteria***" means the one or more criteria that the Committee (or, to the extent that an Award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Board or the Committee) will select for purposes of establishing the Performance Goals for a Performance Period. The Performance Criteria that will be used to establish such Performance Goals may be based on any one of, or combination of, the following as determined by the Committee (or Board, if applicable): (i) earnings (including earnings per share and net earnings); (ii) earnings before interest, taxes and depreciation; (iii) earnings before interest, taxes, depreciation and amortization; (iv) earnings before interest, taxes, depreciation, amortization and legal settlements; (v) earnings before interest, taxes, depreciation, amortization, legal settlements and other income (expense); (vi) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense) and stock-based compensation; (vii) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation and changes in deferred revenue; (viii) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation, other non-cash expenses and changes in deferred revenue; (ix) total shareholder return; (x) return on equity or average

shareholder's equity; (xi) return on assets, investment, or capital employed; (xii) stock price; (xiii) margin (including gross margin); (xiv) income (before or after taxes); (xv) operating income; (xvi) operating income after taxes; (xvii) pre-tax profit; (xviii) operating cash flow; (xix) sales or revenue targets; (xx) increases in revenue or product revenue; (xxi) expenses and cost reduction goals; (xxii) improvement in or attainment of working capital levels; (xxiii) economic value added (or an equivalent metric); (xxiv) market share; (xxv) cash flow; (xxvi) cash flow per share; (xxvii) cash balance; (xxviii) cash burn; (xxix) cash collections; (xxx) share price performance; (xxxi) debt reduction; (xxxii) implementation or completion of projects or processes (including, without limitation, clinical trial initiation, clinical trial enrollment and dates, clinical trial results, regulatory filing submissions, regulatory filing acceptances, regulatory or advisory committee interactions, regulatory approvals, and product supply); (xxxiii) shareholders' equity; (xxxiv) capital expenditures; (xxxv) debt levels; (xxxvi) operating profit or net operating profit; (xxxvii) workforce diversity; (xxxviii) growth of net income or operating income; (xxxix) billings; (xl) bookings; (xli) employee retention; (xlii) initiation of studies by specific dates; (xliii) budget management; (xliv) submission to, or approval by, a regulatory body (including, but not limited to the U.S. Food and Drug Administration) of an applicable filing or a product; (xlv) regulatory milestones; (xlvi) progress of internal research or development programs; (xlvii) acquisition of new customers; (xlviii) customer retention and/or repeat order rate; (xlix) improvements in sample and test processing times; (l) progress of partnered programs; (li) partner satisfaction; (lii) timely completion of clinical trials; (liii) submission of 510(k)s or pre-market approvals and other regulatory achievements; (liv) milestones related to research development (including, but not limited to, preclinical and clinical studies), product development and manufacturing; (lv) expansion of sales in additional geographies or markets; (lvi) research progress, including the development of programs; (lvii) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property; and (lviii) and to the extent that an Award is not intended to comply with Section 162(m) of the Code, other measures of performance selected by the Board or the Committee.

(nn) "***Performance Goals***" means, for a Performance Period, the one or more goals established by the Committee (or, to the extent that an Award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Board or the Committee) for the Performance Period based upon the Performance Criteria. Performance Goals may be based on a Company-wide basis, with respect to one or more business units, divisions, Affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. The Committee (or, to the extent that an Award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Board or the Committee) is authorized to make appropriate adjustments in the method of calculating the attainment of Performance Goals for a Performance Period as follows; *provided, however*, that to the extent that an Award is intended to qualify as "performance-based compensation" under Section 162(m) of the Code, any such adjustment may be made only if such adjustment is objectively determinable and specified in the Award Agreement at the time the Award is granted or in such other document setting forth the Performance Goals for the Award at the time the Performance Goals are established: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of any items that are "unusual" in nature or occur "infrequently" as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by the Company achieved performance objectives at targeted levels during the balance of a Performance Period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of common stock of the Company by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common shareholders other than regular cash dividends; (9) to exclude the effects of stock based compensation and the award of bonuses under the Company's bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles; (12) to exclude the effects of the timing of acceptance for review and/or approval of submissions to the U.S. Food and Drug Administration or any other regulatory body; and (13) to the extent that an Award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, to make other appropriate adjustments selected by the Board or the Committee.

(oo) "***Performance Period***" means the period of time selected by the Committee (or, to the extent that an Award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Board or the Committee) over which the attainment of one or more Performance Goals will be measured for the purpose of

determining a Participant's right to and the payment of a Stock Award or a Performance Cash Award. Performance Periods may be of varying and overlapping duration, at the sole discretion of the Committee (or Board, if applicable).

(pp) "***Performance Stock Award***" means a Stock Award granted under the terms and conditions of Section 6(c)(i).

(qq) "***Plan***" means this BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan.

(rr) "***Restricted Stock Award***" means an award of shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(a).

(ss) "***Restricted Stock Award Agreement***" means a written agreement between the Company and a holder of a Restricted Stock Award evidencing the terms and conditions of a Restricted Stock Award grant. Each Restricted Stock Award Agreement will be subject to the terms and conditions of the Plan.

(tt) "***Restricted Stock Unit Award***" means a right to receive shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(b).

(uu) "***Restricted Stock Unit Award Agreement***" means a written agreement between the Company and a holder of a Restricted Stock Unit Award evidencing the terms and conditions of a Restricted Stock Unit Award grant. Each Restricted Stock Unit Award Agreement will be subject to the terms and conditions of the Plan.

(vv) "***Rule 16b-3***" means Rule 16b-3 promulgated under the Exchange Act or any successor to Rule 16b-3, as in effect from time to time.

(ww) "***Securities Act***" means the Securities Act of 1933, as amended.

(xx) "***Stock Appreciation Right***" or "***SAR***" means a right to receive the appreciation on Common Stock that is granted pursuant to the terms and conditions of Section 5.

(yy) "***Stock Appreciation Right Agreement***" means a written agreement between the Company and a holder of a Stock Appreciation Right evidencing the terms and conditions of a Stock Appreciation Right grant. Each Stock Appreciation Right Agreement will be subject to the terms and conditions of the Plan.

(zz) "***Stock Award***" means any right to receive Common Stock granted under the Plan, including an Incentive Stock Option, a Nonstatutory Stock Option, a Restricted Stock Award, a Restricted Stock Unit Award, a Stock Appreciation Right, a Performance Stock Award or any Other Stock Award.

(aaa) "***Stock Award Agreement***" means a written agreement between the Company and a Participant evidencing the terms and conditions of a Stock Award grant. Each Stock Award Agreement will be subject to the terms and conditions of the Plan.

(bbb) "***Subsidiary***" means, with respect to the Company, (i) any corporation of which more than 50% of the outstanding capital stock having ordinary voting power to elect a majority of the board of directors of such corporation (irrespective of whether, at the time, stock of any other class or classes of such corporation will have or might have voting power by reason of the happening of any contingency) is at the time, directly or indirectly, Owned by the Company, and (ii) any partnership, limited liability company or other entity in which the Company has a direct or indirect interest (whether in the form of voting or participation in profits or capital contribution) of more than 50%.

(ccc) *"**Substitute Award**"* means an Award issued in connection with a merger or acquisition in connection with the assumption of, or substitution for, an existing award.

(ddd) *"**Ten Percent Shareholder**"* means a person who Owns (or is deemed to Own pursuant to Section 424(d) of the Code) stock possessing more than 10% of the total combined voting power of all classes of stock of the Company or any Affiliate.

(eee) *"**Transaction**"* means a Corporate Transaction or a Change in Control.

Exhibit 31.1

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.

August 2, 2019

/s/ JEAN-JACQUES BIENAIMÉ

Jean-Jacques Bienaimé
Chief Executive Officer

Exhibit 31.2

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.

August 2, 2019

/s/ DANIEL SPIEGELMAN

Daniel Spiegelman
Executive Vice President and Chief Financial Officer

Exhibit 32.1

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 June 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

August 2, 2019

/s/ DANIEL SPIEGELMAN

Daniel Spiegelman
Executive Vice President and Chief Financial Officer

August 2, 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.