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, 2024

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: 190,382,822 shares of common stock, par value $0.001, outstanding as of July 31, 2024.

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

BioMarin®, BRINEURA®, KUVAN®, NAGLAZYME®, PALYNZIQ®, ROCTAVIAN®, VIMIZIM® and VOXZOGO® are our registered trademarks. ALDURAZYME® is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this report are the property of their respective owners.

Forward-Looking Statements

This Quarterly Report on Form 10-Q contains "forward-looking statements" as defined under securities laws. Many of these statements can be identified by the use of terminology such as "believes," "expects," "intends," "anticipates," "plans," "may," "will," "could," "would," "projects," "continues," "estimates," "potential," "opportunity" or the negative versions of these terms and other similar expressions. Our actual results or experience could differ significantly from the forward-looking statements. Factors that could cause or contribute to these differences include those discussed in "Risk Factors," in Part II, Item 1A of this Quarterly Report on Form 10-Q as well as information provided elsewhere in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2023, which was filed with the Securities and Exchange Commission (the SEC) on February 26, 2024. 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.

Risk Factors Summary

The following is a summary of the principal risks that could adversely affect our business, financial condition, operating results, cash flows or stock price. Discussion of the risks listed below, and other risks that we face, are discussed in the section titled "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q.

Business and Operational Risks

- If we fail to obtain and maintain 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.
- As compared to our other, more traditional products, gene therapy products may present additional challenges with respect to the pricing, coverage, reimbursement, and acceptance of the product.
- Because the target patient populations for our products are relatively small, we must achieve significant market share and maintain high per-patient prices for our products to achieve and maintain profitability.
- 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 revenues could be adversely affected.
- Changes in methods of treatment of disease could reduce demand for our products and adversely affect revenues.
- 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.
- The sale of generic versions of KUVAN by generic manufacturers has adversely affected and will continue to adversely affect our revenues and may cause a decline in KUVAN revenues faster than expected.
- If we do not achieve our projected development goals in the timeframes we announce or fail to achieve such goals, the commercialization of our product candidates may be delayed or never occur and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

Regulatory Risks

- 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 revenues 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.
- 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 U.S. Food and Drug Administration (FDA), the European Commission (EC), the European Medicines Agency (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 revenues 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.
- To obtain regulatory approval to market our products, preclinical studies and costly and lengthy clinical trials are required and the results of the studies and trials are highly uncertain. Likewise, preliminary, initial or interim data from clinical trials should be considered carefully and with caution because the final data may be materially different from the preliminary, initial or interim data, particularly as more patient data become available.
- 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 revenues and results of operations.
- Government healthcare reform could increase our costs and adversely affect our revenues and results of operations.

Financial and Financing Risks

- If we incur operating losses or are unable to sustain positive cash flows for a period longer than anticipated, we may be unable to continue our operations at planned levels and may be forced to reduce our operations.

Manufacturing Risks

- If we fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.
- If we are unable to successfully develop and maintain manufacturing processes for our product candidates to produce sufficient quantities at acceptable costs, we may be unable to support a clinical trial or be forced to terminate a program, or if we are unable to produce sufficient quantities of our products at acceptable costs, we may be unable to meet commercial demand, lose potential revenue, have reduced margins or be forced to terminate a program.
- 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.

Risks Related to International Operations

- We conduct a significant amount of our operations and generate a significant percentage of our sales outside of the U.S., which subjects us to additional business risks that could adversely affect our revenues and results of operations.
- 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 revenues in these countries.
- Our international operations pose currency risks, which may adversely affect our operating results and net income.

Intellectual Property Risks

- If we are unable to protect our intellectual property, we may not be able to compete effectively or preserve our market shares.
- 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.

BIOMARIN PHARMACEUTICAL INC.

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME

Three and Six Months Ended June 30, 2024 and 2023
(In thousands, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
REVENUES:				
Net product revenues	$ 702,129	$ 584,698	$ 1,339,944	$ 1,171,124
Royalty and other revenues	9,900	10,577	20,918	20,566
Total revenues	712,029	595,275	1,360,862	1,191,690
OPERATING EXPENSES:				
Cost of sales	130,459	130,619	255,639	266,091
Research and development	183,787	177,363	388,774	349,209
Selling, general and administrative	263,032	206,103	488,938	417,126
Intangible asset amortization	14,299	15,624	28,597	31,294
Gain on sale of nonfinancial assets	—	—	(10,000)	—
Total operating expenses	591,577	529,709	1,151,948	1,063,720
INCOME FROM OPERATIONS	120,452	65,566	208,914	127,970
Interest income	19,785	12,612	39,150	24,555
Interest expense	(3,574)	(3,755)	(7,121)	(7,458)
Other expense, net	(4,527)	(3,613)	(3,260)	(17,500)
INCOME BEFORE INCOME TAXES	132,136	70,810	237,683	127,567
Provision for income taxes	24,962	14,770	41,847	20,675
NET INCOME	$ 107,174	$ 56,040	$ 195,836	$ 106,892
EARNINGS PER SHARE, BASIC	$ 0.56	$ 0.30	$ 1.03	$ 0.57
EARNINGS PER SHARE, DILUTED	$ 0.55	$ 0.29	$ 1.01	$ 0.56
Weighted average common shares outstanding, basic	190,114	187,948	189,490	187,311
Weighted average common shares outstanding, diluted	200,505	194,998	200,137	194,756
COMPREHENSIVE INCOME	$ 135,019	$ 48,145	$ 251,423	$ 92,142

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

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

June 30, 2024 and December 31, 2023

(In thousands, except share amounts)

	June 30, 2024	December 31, 2023 [1]
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 972,150	$ 755,127
Short-term investments	252,201	318,683
Accounts receivable, net	691,232	633,704
Inventory	1,183,621	1,107,183
Other current assets	160,426	141,391
Total current assets	3,259,630	2,956,088
Noncurrent assets:		
Long-term investments	557,083	611,135
Property, plant and equipment, net	1,052,898	1,066,133
Intangible assets, net	265,533	294,701
Goodwill	196,199	196,199
Deferred tax assets	1,545,006	1,545,809
Other assets	190,772	171,538
Total assets	$ 7,067,121	$ 6,841,603
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 572,500	$ 683,147
Short-term convertible debt, net	494,837	493,877
Total current liabilities	1,067,337	1,177,024
Noncurrent liabilities:		
Long-term convertible debt, net	594,116	593,095
Other long-term liabilities	119,369	119,935
Total liabilities	1,780,822	1,890,054
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 190,355,517 and 188,598,154 shares issued and outstanding, respectively	190	189
Additional paid-in capital	5,696,701	5,611,562
Company common stock held by the Nonqualified Deferred Compensation Plan	(11,673)	(9,860)
Accumulated other comprehensive income (loss)	26,799	(28,788)
Accumulated deficit	(425,718)	(621,554)
Total stockholders' equity	5,286,299	4,951,549
Total liabilities and stockholders' equity	$ 7,067,121	$ 6,841,603

(1) December 31, 2023 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, 2023, filed with the SEC on February 26, 2024.

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

BIOMARIN PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
Three and Six Months Ended June 30, 2024 and 2023
(In thousands)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Shares of common stock, beginning balances [1]	189,777	187,601	188,598	186,251
Issuances under equity incentive plans	579	551	1,758	1,901
Shares of common stock, ending balances	190,356	188,152	190,356	188,152
Total stockholders' equity, beginning balances [1]	$ 5,073,816	$ 4,659,043	$ 4,951,549	$ 4,603,156
Common stock:				
Beginning balances [1]	190	188	189	186
Issuances under equity incentive plans, net of tax	—	—	1	2
Ending balances	190	188	190	188
Additional paid-in capital:				
Beginning balances [1]	5,619,264	5,417,873	5,611,562	5,404,895
Issuances under equity incentive plans, net of tax	25,326	24,489	(30,343)	(17,951)
Stock-based compensation	52,138	51,150	113,669	105,478
Change in Common stock held by the Nonqualified Deferred Compensation plan (NQDC)	(27)	444	1,813	1,534
Ending balances	5,696,701	5,493,956	5,696,701	5,493,956
Company common stock held by the NQDC:				
Beginning balances [1]	(11,700)	(9,949)	(9,860)	(8,859)
Common stock held by the NQDC	27	(444)	(1,813)	(1,534)
Ending balances	(11,673)	(10,393)	(11,673)	(10,393)
Accumulated other comprehensive income (loss):				
Beginning balances [1]	(1,046)	(10,722)	(28,788)	(3,867)
Other comprehensive income (loss)	27,845	(7,895)	55,587	(14,750)
Ending balances	26,799	(18,617)	26,799	(18,617)
Accumulated Deficit:				
Beginning balances [1]	(532,892)	(738,347)	(621,554)	(789,199)
Net income	107,174	56,040	195,836	106,892
Ending balances	(425,718)	(682,307)	(425,718)	(682,307)
Total stockholders' equity, ending balances	$ 5,286,299	$ 4,782,827	$ 5,286,299	$ 4,782,827

(1) The beginning balances for the six-month periods 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, 2023 filed with the SEC on February 26, 2024.

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, 2024 and 2023
(In thousands)
(unaudited)

	Six Months Ended June 30,	
	2024	**2023**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net income	$ 195,836	$ 106,892
Adjustments to reconcile net income to net cash used in operating activities:		
Depreciation and amortization	53,813	51,840
Non-cash interest expense	1,981	2,058
Accretion of discount on investments	(4,678)	(4,533)
Stock-based compensation	106,163	103,857
Gain on sale of nonfinancial assets	(10,000)	—
Impairment of assets and other non-cash adjustments	14,204	12,650
Deferred income taxes	1,537	(5,108)
Unrealized foreign exchange loss (gain)	(19,958)	7,455
Other	(858)	361
Changes in operating assets and liabilities:		
Accounts receivable, net	(56,081)	(145,831)
Inventory	(47,409)	(56,476)
Other current assets	1,615	(53,430)
Other assets	(22,880)	(5,616)
Accounts payable and other short-term liabilities	(54,261)	(25,093)
Other long-term liabilities	6,709	7,104
Net cash provided by (used in) operating activities	165,733	(3,870)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(47,431)	(46,039)
Maturities and sales of investments	317,649	491,063
Purchases of investments	(195,462)	(444,049)
Proceeds from sale of nonfinancial assets	10,000	—
Purchase of intangible assets	(8,512)	(1,457)
Net cash provided by (used in) investing activities	76,244	(482)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of awards under equity incentive plans	36,618	50,193
Taxes paid related to net share settlement of equity awards	(66,739)	(67,862)
Payments of contingent consideration	—	(9,475)
Principal repayments of financing leases	(60)	(1,635)
Net cash used in financing activities	(30,181)	(28,779)
Effect of exchange rate changes on cash	5,227	2,981
NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	217,023	(30,150)
Cash and cash equivalents:		
Beginning of period	$ 755,127	$ 724,531
End of period	$ 972,150	$ 694,381
SUPPLEMENTAL CASH FLOW DISCLOSURES:		
Cash paid for interest	$ 5,126	$ 5,175
Cash paid for income taxes	$ 22,884	$ 28,183
SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON-CASH INVESTING AND FINANCING ACTIVITIES:		
Increase in accounts payable and accrued liabilities related to fixed assets	$ 11,941	$ 8,669
Decrease in accounts payable and accrued liabilities related to intangible assets	$ 8,290	$ 2,344

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

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

(1) BUSINESS OVERVIEW AND SIGNIFICANT ACCOUNTING POLICIES

Nature of Operations

Founded in 1997, BioMarin Pharmaceutical Inc. (the Company or BioMarin) is a global biotechnology company dedicated to transforming lives through genetic discovery. The Company develops and commercializes targeted therapies that address the root cause of genetic conditions. The Company's robust research and development (R&D) capabilities have resulted in multiple innovative commercial therapies for patients with rare genetic disorders. The Company's distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options.

Basis of Presentation

These Condensed Consolidated Financial Statements have been prepared pursuant to 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 management 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, 2023 included in the Company’s Annual Report on Form 10-K. The Condensed Consolidated Financial Statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany transactions have been eliminated. The results of operations for the three and six months ended June 30, 2024 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2024 or any other period.

Change in Presentation

Effective January 1, 2024, the Company changed its presentation for foreign currency transaction gains and losses resulting from remeasurement and idle plant costs within its Condensed Consolidated Statements of Comprehensive Income. Effective with this change in presentation, foreign currency transaction gains and losses resulting from remeasurement are presented in Other Expense, Net and idle plant costs are presented in Cost of Sales. Prior to this change in presentation, both foreign currency transaction gains and losses resulting from remeasurement and idle plant costs were presented in Selling, General and Administrative (SG&A) expense. The Company believes that this change in presentation is preferable because the revised presentation is more consistent with how management measures the Company’s operating performance.

Prior period amounts were revised to conform to current period presentation. The following table reflects the impacts of the change in presentation for the prior periods presented. The change in presentation had no impact to Net Income, Total Stockholders’ Equity or earnings per share for the three and six months ended June 30, 2023.

	Three Months ended June 30, 2023	Six Months ended June 30, 2023
Cost of sales	$ 2,537	$ 11,460
SG&A	$ (9,233)	$ (21,213)
Total operating expenses	$ (6,696)	$ (9,753)
Other expense, net	$ 6,696	$ 9,753

Use of Estimates

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 to determine if there were any subsequent events that occurred subsequent to the balance sheet date and prior to filing this Quarterly Report on Form 10-Q, that would require recognition or disclosure in the Condensed Consolidated Financial Statements. Further to this evaluation, the Company has included a subsequent event noted relating to the maturity and settlement of the Company’s convertible notes due in August 2024 that is discussed in *Note 6 - Debt*.

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

Significant Accounting Policies

There have been no material changes to the Company's significant accounting policies during the six months ended June 30, 2024, as compared to the significant accounting policies disclosed in Note 1 – *Business Overview and Significant Accounting Policies* to the Company's Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023.

Recent Accounting Pronouncements

There have been no new accounting pronouncements adopted by the Company or new accounting pronouncements issued by the Financial Accounting Standards Board (FASB) during the six months ended June 30, 2024, as compared to the recent accounting pronouncements described in Note 1 to the Company's Consolidated Financial Statements of the Company's Annual Report on Form 10-K for the year ended December 31, 2023, that the Company believes are of significance or potential significance to the Company. The following paragraphs discuss new accounting pronouncements issued by the FASB, but not yet adopted by the Company.

In November 2023, the FASB issued Accounting Standards Update (ASU) 2023-07, Segment Reporting Topic 280, *Improvements to Reportable Segment Disclosures*, to improve reportable segment disclosure requirements through enhanced disclosures about significant segment expenses. ASU 2023-07 expands public entities' segment disclosures by requiring disclosure of significant segment expenses that are regularly provided to the chief operating decision maker and included within each reported measure of segment profit or loss, an amount and description of its composition for other segment items and interim disclosures of a reportable segment's profit or loss and assets. The effective date for the update is for fiscal years beginning after December 15, 2023 and interim periods within fiscal years beginning after December 15, 2024 and should be applied on a retrospective basis to all periods presented. The Company is currently evaluating the effect of adopting the update on its related disclosures.

In December 2023, the FASB issued ASU 2023-09, Income Taxes Topic 740, *Improvements to Income Tax Disclosures*. The guidance requires disclosure of disaggregated information about the Company's effective tax rate reconciliation as well as information on income taxes paid. The disclosure requirements will be applied on a prospective basis, with the option to apply it retrospectively. The effective date for the update is for fiscal years beginning after December 15, 2024. The Company is currently evaluating the effect of the update on its related disclosures.

(2) FINANCIAL INSTRUMENTS

All marketable securities were classified as available-for-sale as of June 30, 2024 and December 31, 2023.

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category for each period presented:

	June 30, 2024						
	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	$ 285,666	$ —	$ —	$ 285,666	$ 285,666	$ —	$ —
Level 2:							
Money market instruments	676,505	—	—	676,505	676,505	—	—
Corporate debt securities	537,626	670	(1,460)	536,836	—	162,959	373,877
U.S. government agency securities	184,487	39	(966)	183,560	9,979	88,310	85,271
Asset-backed securities	98,958	105	(196)	98,867		932	97,935
Subtotal	1,497,576	814	(2,622)	1,495,768	686,484	252,201	557,083
Total	$ 1,783,242	$ 814	$ (2,622)	$ 1,781,434	$ 972,150	$ 252,201	$ 557,083

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

	December 31, 2023						
	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	$ 229,676	$ —	$ —	$ 229,676	$ 229,676	$ —	$ —
Level 2:							
Money market instruments	499,483	—	—	499,483	499,483	—	—
Corporate debt securities	587,896	3,476	(1,996)	589,376	—	193,251	396,125
U.S. government agency securities	251,952	556	(1,140)	251,368	19,976	111,343	120,049
Commercial paper	20,076	5	—	20,081	5,992	14,089	—
Asset-backed securities	94,744	351	(134)	94,961	—	—	94,961
Subtotal	1,454,151	4,388	(3,270)	1,455,269	525,451	318,683	611,135
Total	$ 1,683,827	$ 4,388	$ (3,270)	$ 1,684,945	$ 755,127	$ 318,683	$ 611,135

(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, 2024, the Company had the ability and intent to hold all investments that were in an unrealized loss position until maturity. The Company considered its intent and ability to hold the securities until recovery of amortized cost basis, the extent to which fair value is less than amortized cost basis, conditions specifically related to the security's industry and geography, payment structure and history and changes to the ratings (if any) in determining that the decline in fair value compared to carrying value is not related to a credit loss.

The Company has certain investments in non-marketable equity securities, measured using unobservable valuation inputs and remeasured on a nonrecurring basis, which are collectively considered strategic investments. As of June 30, 2024 and December 31, 2023, the fair value of the Company's strategic investments was $6.7 million and $11.3 million, respectively. These investments were recorded to Other Assets in the Company's Condensed Consolidated Balance Sheets. In the second quarter of 2024, based on new developments, the Company became aware of factors that indicated a $4.5 million decline in the fair value of one of its strategic investments. In the first quarter of 2023, the Company concluded that factors existed indicating it would no longer realize a $12.6 million equity investment in its non-marketable securities. The losses on the Company's non-marketable equity investments were recorded to Other Expense, Net on the Company's Condensed Consolidated Statements of Comprehensive Income for the respective periods. See Note 1 - *Business Overview and Significant Accounting Policies* included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023 for additional information related to the Company's non-marketable securities policy.

(3) SUPPLEMENTAL FINANCIAL STATEMENTS INFORMATION

Supplemental Balance Sheet Information

Inventory consisted of the following:

	June 30, 2024	December 31, 2023
Raw materials	$ 154,153	$ 155,704
Work-in-process	605,770	571,107
Finished goods	423,698	380,372
Total inventory	$ 1,183,621	$ 1,107,183

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

Property, Plant and Equipment, Net consisted of the following:

	June 30, 2024	December 31, 2023
Property, plant and equipment, gross	$ 1,962,948	$ 1,933,222
Accumulated depreciation	(910,050)	(867,089)
Total property, plant and equipment, net	$ 1,052,898	$ 1,066,133

Depreciation expense, net of amounts capitalized into inventory, for the three and six months ended June 30, 2024 was $11.5 million and $24.1 million, respectively. Depreciation expense, net of amounts capitalized into inventory, for the three and six months ended June 30, 2023 was $9.4 million and $19.7 million, respectively.

Intangible Assets, Net consisted of the following:

	June 30, 2024	December 31, 2023
Finite-lived intangible assets	$ 710,533	$ 710,011
Accumulated amortization	(445,000)	(415,310)
Net carrying value	$ 265,533	$ 294,701

In the first quarter of 2024, the Company received $10.0 million due to the achievement of a regulatory approval milestone by a third party related to previously sold intangible assets, which the Company recorded as a Gain on Sale of Nonfinancial Assets in the Condensed Consolidated Statements of Comprehensive Income.

Accounts Payable and Accrued Liabilities consisted of the following:

	June 30, 2024	December 31, 2023
Accounts payable and accrued operating expenses	$ 257,412	$ 315,509
Accrued compensation expense	147,737	201,067
Accrued rebates payable	113,432	96,179
Accrued income taxes	18,864	2,651
Lease liability	10,689	8,779
Foreign currency exchange forward contracts	7,684	33,853
Accrued royalties payable	7,667	14,299
Deferred revenue	1,912	4,620
Other	7,103	6,190
Total accounts payable and accrued liabilities	$ 572,500	$ 683,147

(4) FAIR VALUE MEASUREMENTS

The Company measures certain financial assets and liabilities at fair value in accordance with the policy described in Note 1 – *Business Overview and Significant Accounting Policies* to the Company's Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023.

Other than the Company's fixed-rate convertible debt disclosed in Note 6 – *Debt*, there were no financial assets or liabilities that were remeasured using quoted prices in active markets for identical assets (Level 1) as of June 30, 2024 or

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

December 31, 2023. The Company had no financial assets or liabilities that are remeasured on a recurring basis using unobservable inputs that reflect estimates and assumptions (Level 3) as of June 30, 2024 or December 31, 2023.

Level 2 assets and liabilities that are remeasured using significant observable inputs consisted of the following:

	June 30, 2024	December 31, 2023
Assets:		
Other current assets:		
NQDC Plan assets	$ 2,649	$ 2,026
Other assets:		
NQDC Plan assets	33,112	28,119
Restricted investments [1]	2,419	2,393
Total other assets	35,531	30,512
Total assets	$ 38,180	$ 32,538
Liabilities:		
Current liabilities:		
NQDC Plan liability	$ 2,649	$ 2,026
Other long-term liabilities:		
NQDC Plan liability	33,112	28,119
Total liabilities	$ 35,761	$ 30,145

(1) The restricted investments as of June 30, 2024 and December 31, 2023 secure the Company's irrevocable standby letters of credit obtained in connection with certain commercial agreements.

There were no transfers between levels during the three and six months ended June 30, 2024.

(5) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

The Company uses foreign currency exchange forward contracts (forward contracts) to protect against the impact of changes in the value of forecasted foreign currency cash flows resulting from revenues and operating expenses denominated in currencies other than the U.S. Dollar (USD), primarily the Euro. Certain of these forward contracts are designated as cash flow hedges and have maturities of up to 24 months. The Company also enters into forward contracts to manage foreign exchange risk related to asset or liability positions denominated in currencies other than USD. Such forward contracts are considered to be economic hedges, are not designated as hedging instruments and have maturities of up to three months. The Company does not use derivative instruments for speculative trading purposes. The Company is exposed to counterparty credit risk on its derivatives. The Company has established and maintains strict counterparty credit guidelines and enters into hedging agreements with financial institutions that are investment grade or better to minimize the Company's exposure to potential defaults. The Company is not required to pledge collateral under these agreements.

The following table summarizes the aggregate notional amounts for the Company's derivatives outstanding as of the periods presented.

Forward Contracts	June 30, 2024	December 31, 2023
Derivatives designated as hedging instruments:		
Sell	$ 1,086,980	$ 1,249,662
Purchase	$ 192,162	$ 198,408
Derivatives not designated as hedging instruments:		
Sell	$ 281,438	$ 350,269
Purchase	$ 28,824	$ 90,102

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

The fair value carrying amounts of the Company's derivatives, which are classified as Level 2 within the fair value hierarchy, were as follows:

Balance Sheet Location	June 30, 2024	December 31, 2023
Derivatives designated as hedging instruments:		
Asset Derivatives		
Other current assets	$ 27,599	$ 6,663
Other assets	8,236	1,855
Subtotal	$ 35,835	$ 8,518
Liability Derivatives		
Accounts payable and accrued liabilities	$ 6,407	$ 30,005
Other long-term liabilities	1,257	8,171
Subtotal	$ 7,664	$ 38,176
Derivatives not designated as hedging instruments:		
Asset Derivatives		
Other current assets	$ 2,352	$ 547
Liability Derivatives		
Accounts payable and accrued liabilities	$ 1,277	$ 3,848
Total Derivatives Assets	$ 38,187	$ 9,065
Total Derivatives Liabilities	$ 8,941	$ 42,024

For additional discussion of fair value measurements, see Note 1 – *Business Overview and Significant Accounting Policies* to the Company's Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023.

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

	Three Months Ended		Six Months Ended June 30,	
	2024	2023	2024	2023
Derivatives Designated as Cash Flow Hedging Instruments	**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**	**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**	**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**	**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**
Net product revenues	$ 3,371	$ 677	$ 2,252	$ 4,147
Operating expenses	$ (190)	$ (26)	$ 314	$ (482)
Derivatives Not Designated as Hedging Instruments	**Gains (Losses) Recognized in Earnings**	**Gains (Losses) Recognized in Earnings**	**Gains (Losses) Recognized in Earnings**	**Gains (Losses) Recognized in Earnings**
Operating expenses	$ 13,812	$ 769	$ 21,541	$ (3,394)

As of June 30, 2024, the Company expects to reclassify unrealized gains of $21.2 million from Accumulated Other Comprehensive Income (Loss) (AOCI) to earnings as the forecasted revenues and operating expense transactions occur over the next twelve months. For additional discussion of balances in AOCI see Note 7 – *Accumulated Other Comprehensive Income*.

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

(6) DEBT

Convertible Notes

As of June 30, 2024, the Company had outstanding fixed-rate notes with varying maturities for an undiscounted aggregate principal amount of $1.1 billion (collectively, the Notes). The Notes are senior subordinated convertible obligations, and interest is payable in arrears, semi-annually. The following table summarizes information regarding the Company's convertible debt:

	June 30, 2024	December 31, 2023
0.599% senior subordinated convertible notes due in August 2024 (the 2024 Notes)	$ 495,000	$ 495,000
Unamortized discount net of deferred offering costs	(163)	(1,123)
2024 Notes, net [1]	494,837	493,877
1.25% senior subordinated convertible notes due in May 2027 (the 2027 Notes)	600,000	600,000
Unamortized discount net of deferred offering costs	(5,884)	(6,905)
2027 Notes, net	594,116	593,095
Total convertible debt, net	$ 1,088,953	$ 1,086,972
Fair value of fixed-rate convertible debt [2]:		
2024 Notes	$ 492,447	$ 488,288
2027 Notes	576,102	619,260
Total fair value of fixed-rate convertible debt	$ 1,068,549	$ 1,107,548

(1) The Company's convertible notes due in 2024 matured on August 1, 2024, subsequent to quarter-end. Substantially all holders of the 2024 Notes were repaid with cash, totaling approximately $495.0 million. No gain or loss was incurred upon the extinguishment.

(2) 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 1 – *Business Overview and Significant Accounting Policies* to the Company's Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Coupon interest expense	$ 2,617	$ 2,617	$ 5,233	$ 5,233
Accretion of discount on convertible notes	843	834	1,684	1,673
Amortization of debt issuance costs	148	149	297	297
Total interest expense on convertible debt	$ 3,608	$ 3,600	$ 7,214	$ 7,203

See Note 10 - *Debt* to the Company's Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023 for additional information related to the Company's convertible debt.

(7) ACCUMULATED OTHER COMPREHENSIVE INCOME

The following tables summarize changes in the accumulated balances for each component of AOCI, including current-period other comprehensive income and reclassifications out of AOCI, for the periods presented.

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

	Three Months Ended June 30, 2024		
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Total
AOCI balance as of March 31, 2024	$ (183)	$ (863)	$ (1,046)
Other comprehensive income (loss) before reclassifications	31,551	(684)	30,867
Less: gain (loss) reclassified from AOCI	3,181	—	3,181
Tax effect	—	159	159
Net current-period other comprehensive income (loss)	28,370	(525)	27,845
AOCI balance as of June 30, 2024	$ 28,187	$ (1,388)	$ 26,799

	Three Months Ended June 30, 2023		
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Total
AOCI balance as of March 31, 2023	$ (2,647)	$ (8,075)	$ (10,722)
Other comprehensive income (loss) before reclassifications	(6,737)	(661)	(7,398)
Less: gain (loss) reclassified from AOCI	651	—	651
Tax effect	—	154	154
Net current-period other comprehensive income (loss)	(7,388)	(507)	(7,895)
AOCI balance as of June 30, 2023	$ (10,035)	$ (8,582)	$ (18,617)

	Six Months Ended June 30, 2024		
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Total
AOCI balance as of December 31, 2023	$ (29,658)	$ 870	$ (28,788)
Other comprehensive income (loss) before reclassifications	60,411	(2,926)	57,485
Less: gain (loss) reclassified from AOCI	2,566	—	2,566
Tax effect	—	668	668
Net current-period other comprehensive income (loss)	57,845	(2,258)	55,587
AOCI balance as of June 30, 2024	$ 28,187	$ (1,388)	$ 26,799

	Six Months Ended June 30, 2023		
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Total
AOCI balance as of December 31, 2022	$ 8,226	$ (12,093)	$ (3,867)
Other comprehensive income (loss) before reclassifications	(14,596)	4,586	(10,010)
Less: gain (loss) reclassified from AOCI	3,665	—	3,665
Tax effect	—	(1,075)	(1,075)
Net current-period other comprehensive income (loss)	(18,261)	3,511	(14,750)
AOCI balance as of June 30, 2023	$ (10,035)	$ (8,582)	$ (18,617)

For additional discussion of reclassifications from AOCI see Note 5 – *Derivative Instruments and Hedging Strategies*.

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

(8) REVENUE, CREDIT CONCENTRATIONS AND GEOGRAPHIC INFORMATION

The Company operates in one business segment, which focuses on the development and commercialization of innovative therapies for people with serious and life-threatening rare diseases and medical conditions.

The following table presents Total Revenues and disaggregates Net Product Revenues by product.

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
VOXZOGO	$ 183,923	$ 113,337	$ 336,812	$ 201,173
VIMIZIM	178,016	177,392	370,536	366,584
NAGLAZYME	132,049	90,103	237,678	213,124
PALYNZIQ	88,291	74,868	164,000	137,220
BRINEURA	45,309	38,058	84,356	77,202
ALDURAZYME	38,558	40,318	73,820	74,721
KUVAN	28,551	50,622	64,461	101,100
ROCTAVIAN	7,432	—	8,281	—
Total net product revenues	702,129	584,698	1,339,944	1,171,124
Royalty and other revenues	9,900	10,577	20,918	20,566
Total revenues	$ 712,029	$ 595,275	$ 1,360,862	$ 1,191,690

The Company considers there to be revenue concentration risks for regions where Net Product Revenues exceed 10% of consolidated Net Product 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 table below disaggregates total Net Product Revenues by geographic region, which is based on patient location for the Company's commercial products sold directly by the Company, except for ALDURAZYME, which is marketed and sold exclusively by Sanofi worldwide.

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
United States	$ 227,449	$ 191,655	$ 420,448	$ 358,439
Europe	220,990	183,434	416,738	344,126
Latin America	84,418	75,444	163,012	143,192
Middle East	49,701	25,630	106,776	117,272
Rest of world	81,013	68,217	159,150	133,374
Total net product revenues marketed by the Company	$ 663,571	$ 544,380	$ 1,266,124	$ 1,096,403
ALDURAZYME net product revenues marketed by Sanofi	38,558	40,318	73,820	74,721
Total net product revenues	$ 702,129	$ 584,698	$ 1,339,944	$ 1,171,124

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,	
	2024	2023	2024	2023
Customer A	14 %	15 %	14 %	15 %
Customer B	11 %	12 %	11 %	11 %
Customer C	10 %	10 %	9 %	9 %
Total	35 %	37 %	34 %	35 %

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

On a consolidated basis, three customers accounted for 15%, 12%, and 10% of the Company's June 30, 2024 accounts receivable balance, respectively, compared to December 31, 2023, when two customers accounted for 15% and 12% of the accounts receivable balance, respectively. As of June 30, 2024, and December 31, 2023, the accounts receivable balance for Sanofi included $67.1 million and $63.4 million, respectively, of unbilled accounts receivable, which becomes payable to the Company when the product is sold through by Sanofi. The Company does not require collateral from its customers, but does perform periodic credit evaluations of its customers' financial condition and requires prepayments in certain circumstances.

The Company is mindful that conditions in the current macroeconomic environment, such as inflation, changes in interest and foreign currency exchange rates, natural disasters, geopolitical instability, and supply chain disruptions, could affect the Company's ability to achieve its goals. In addition, the Company sells its products in countries that face economic volatility and weakness. Although the Company has historically collected receivables from customers in certain countries, sustained weakness or further deterioration of the local economies and currencies may cause customers in those countries to delay payment or be unable to pay for the Company's products. The Company believes that the allowances for doubtful accounts related to these countries, if any, are adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. The Company will continue to monitor these conditions and will attempt to adjust its business processes, as appropriate, to mitigate macroeconomic risks to its business.

(9) STOCK-BASED COMPENSATION

The Company has stockholder-approved equity incentive plans that provide for the granting of restricted stock units (RSUs) and stock options as well as other forms of equity compensation to its employees, officers and non-employee directors. The Company also has an Employee Stock Purchase Plan (ESPP). Compensation expense included in the Company's Condensed Consolidated Statements of Comprehensive Income for all stock-based compensation arrangements was as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Cost of sales	$ 3,774	$ 4,721	$ 7,018	$ 9,052
Research and development	12,935	15,055	33,612	34,883
Selling, general and administrative	31,205	30,386	65,533	59,922
Total stock-based compensation expense	$ 47,914	$ 50,162	$ 106,163	$ 103,857

(10) RESTRUCTURING

During the first half of 2024, the Company completed a strategic portfolio assessment of its research and development programs to determine which have the most transformative potential for patients and value creation for shareholders. With the combined focus on patient impact and commercial opportunity, certain programs that met the highest bar for advancement have been prioritized. As a result of the assessment, certain programs have been discontinued.

During the second quarter of 2024, in connection with the discontinuation of certain research and development programs, the Company committed to a plan to reduce its global workforce by approximately 170 employees (representing approximately 5% of the Company's global workforce). Workforce reductions are expected to be substantially completed by end of 2024.

The restructuring plan includes severance and employee-related costs, asset impairments, and other costs. The asset impairment charges were for abandoned assets-in-progress and a Right-of-Use asset (ROU Asset) related to leased office space the Company decided to exit and sub-lease. The Company utilized the discounted cash flow approach to determine the fair value of the ROU Asset. The ROU Asset impairment is the difference between the existing lease terms and rates and the expected sub-lease terms and rates available in the market. The Other category includes restructuring-related costs, which are expensed as incurred, as well as other obligations related to the leased office space that will be satisfied over the remainder of the lease term.

The restructuring charges and adjustments were included in Selling, General, and Administrative in the Condensed Consolidated Statements of Comprehensive Income. Restructuring expenses consisted of the following:

	Three Months Ended June 30,	Six Months Ended June 30,
	2024	2024
Severance and one-time employee benefits	$ 23,837	$ 25,275
Asset Impairments	9,671	9,671
Other	5,519	7,519
	$ 39,027	$ 42,465

The following unpaid balance as of June 30, 2024 was recorded to Accounts Payable and Accrued Liabilities on the Condensed Consolidated Balance Sheet:

	Severance and related costs	Other	Total
Balance as of December 31, 2023	$ —	$ —	$ —
Charges and Adjustments	25,275	7,519	$ 32,793
Payments	(4,321)	(2,000)	$ (6,321)
Balance as of June 30, 2024	$ 20,954	$ 5,519	$ 26,472

(11) EARNINGS 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 ESPP, unvested RSUs and contingent issuances of common stock related to the Company's convertible debt.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Numerator:				
Net Income, basic	$ 107,174	$ 56,040	$ 195,836	$ 106,892
Add: Interest expense, net of tax, on the Company's convertible debt	2,769	937	5,538	1,873
Net Income, diluted	$ 109,943	$ 56,977	$ 201,374	$ 108,765
Denominator:				
Weighted-average common shares outstanding, basic	190,114	187,948	189,490	187,311
Effect of dilutive securities:				
Common stock issuable under the Company's equity incentive plans	2,056	3,080	2,312	3,475
Common stock issuable under the Company's convertible debt[(1)]	8,335	3,970	8,335	3,970
Weighted-average common shares outstanding, diluted	200,505	194,998	200,137	194,756
Earnings per common share, basic	$ 0.56	$ 0.30	$ 1.03	$ 0.57
Earnings per common share, diluted	$ 0.55	$ 0.29	$ 1.01	$ 0.56

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Common stock issuable under the Company's equity incentive plans	10,362	8,716	10,106	8,321
Common stock issuable under the Company's convertible debt [1]	—	4,365	—	4,365
Total number of potentially issuable shares	10,362	13,081	10,106	12,686

(1) If converted, the Company would issue 4.0 million shares under the 2024 Notes and 4.4 million shares under the 2027 Notes. For additional discussion of the Company's convertible debt, see Note 6 - *Debt.*

(12) 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 or 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 based on existing information. The Company accrues for the best estimate of a loss within a range; however, if no estimate in the range is better than any other, then the minimum amount in the range is accrued. Liabilities are evaluated and refined each reporting period as additional information is known. Any receivables for insurance recoveries for these liability claims are recorded as assets when it is probable that a recovery will be realized.

As previously disclosed, the Company received a subpoena from the U.S. Department of Justice (DOJ) requesting that the Company produce certain documents regarding sponsored testing programs relating to VIMIZIM and NAGLAZYME. The Company has produced the requested documents in response to the subpoena and is cooperating fully. The Company is unable to make any assurances regarding the outcome of the investigation by the DOJ, or the impact, if any, that such investigation may have on the Company's business, Condensed Consolidated Balance Sheets, Condensed Consolidated Statements of Comprehensive Income or Condensed Consolidated Statements of Cash Flows.

Contingent Payments

As of June 30, 2024, the Company was subject to contingent payments, primarily comprised of development, regulatory and commercial milestones. Those considered reasonably possible totaled $495.6 million, of this amount the Company may pay up to $10.9 million over the next 12 months if certain contingencies are met.

Other Commitments

The Company uses experts and laboratories at universities and other institutions to perform certain R&D activities. These amounts are recorded as R&D expense as services are provided. In the normal course of business, the Company enters into various firm purchase commitments primarily to procure active pharmaceutical ingredients, certain inventory-related items and certain third-party R&D services, production services and facility construction services. The Company also has commitments related to enterprise resource planning (ERP) system implementation costs for which the Company is committed. As of June 30, 2024, such commitments were estimated at $473.0 million, of which $291.1 million is expected to be paid in 2024 as underlying goods and services are received. The Company has also licensed technology from third parties, for which it is required to pay royalties upon future sales, subject to certain annual minimums.

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 of 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. Our Condensed Consolidated Financial Statements have been prepared in accordance with United States (U.S.) generally accepted accounting principles (U.S. GAAP) and are presented in U.S. Dollars (USD).

(In millions, except as otherwise disclosed)

Overview

Founded in 1997, we are a global biotechnology company dedicated to transforming lives through genetic discovery. We develop and commercialize targeted therapies that address the root cause of genetic conditions. Our robust research and development capabilities have resulted in multiple innovative commercial therapies for patients with rare genetic disorders. Our distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options. A summary of our commercial products, as of June 30, 2024, is provided below:

Commercial Products	Indication
VOXZOGO (vosoritide)	Achondroplasia
VIMIZIM (elosulfase alpha)	Mucopolysaccharidosis (MPS) IVA
NAGLAZYME (galsulfase)	MPS VI
PALYNZIQ (pegvaliase-pqpz)	Phenylketonuria (PKU)
BRINEURA (cerliponase alfa)	Neuronal ceroid lipofuscinosis type 2 (CLN2)
ALDURAZYME (laronidase)	MPS I
KUVAN (sapropterin dihydrochloride)	PKU
ROCTAVIAN (valoctocogene roxaparvovec)	Severe Hemophilia A

Financial Highlights

Key components of our results of operations include the following:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Total revenues	$ 712.0	$ 595.3	$ 1,360.9	$ 1,191.7
Cost of sales	$ 130.5	$ 130.6	$ 255.6	$ 266.1
Research and development (R&D)	$ 183.8	$ 177.4	$ 388.8	$ 349.2
Selling, general and administrative (SG&A)	$ 263.0	$ 206.1	$ 488.9	$ 417.1
Net income	$ 107.2	$ 56.0	$ 195.8	$ 106.9

See "Results of Operations" below for discussion of our results for the periods presented.

Uncertainty Relating to Macroeconomic Environment

Conditions in the current macroeconomic environment, such as inflation, changes in interest and foreign currency exchange rates, natural disasters, geopolitical instability, and supply chain disruptions, could impact our global revenue sources and our overall business operations. The extent and duration of such effects remain uncertain and difficult to predict. We are actively monitoring and managing our response and assessing actual and potential impacts to our operating results and financial condition, as well as developments in our business, which could further impact the developments, trends and expectations described below. See the risk factor, "Our business is affected by macroeconomic conditions." described in "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q.

Recent Developments

We continued to grow our commercial business and advance our product candidate pipeline during the first half of 2024. We believe that the combination of our internal research programs 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.

In the first half of 2024, we focused on value creation through working to accelerate growth, optimize efficiencies and drive operational excellence, including progress in executing on key strategic priorities first outlined in January 2024. We also completed a strategic portfolio assessment of R&D programs to determine which have the most transformative potential for patients and value creation for shareholders and have shifted focus and resources to those R&D programs that met our highest bar for prioritized

(In millions, except as otherwise disclosed)

advancement. The programs, namely BMN 333, a long-acting C-type natriuretic peptide for multiple growth disorders, BMN 349, a potential best-in-class, oral therapeutic for liver disease associated with Alpha-1 Antitrypsin Deficiency, and BMN 351, our next generation oligonucleotide for Duchenne Muscular Dystrophy, all met the highest bar for advancement. Based on such strategic portfolio assessment, certain programs have also been discontinued. In addition, we initiated the execution of our financial and operational strategy that improved operating results and is expected to continue to improve our results of operations. In August 2024, we announced our ROCTAVIAN strategy, which we anticipate will enable ROCTAVIAN to contribute to our long-term profitability. See the risk factor, "Our success depends on our ability to manage our growth." described in "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q.

Change in Presentation

On January 1, 2024, we changed our presentation of foreign currency transaction gains and losses resulting from remeasurement and idle plant costs within our Condensed Consolidated Statements of Comprehensive Income. See Note 1 to our accompanying Condensed Consolidated Financial Statements for additional details.

Results of Operations

Net Product Revenues

Net Product Revenues consisted of the following:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
VOXZOGO	$ 183.9	$ 113.3	$ 70.6	$ 336.7	$ 201.2	$ 135.5
VIMIZIM	178.0	177.4	0.6	370.5	366.6	3.9
NAGLAZYME	132.0	90.1	41.9	237.7	213.1	24.6
PALYNZIQ	88.3	74.9	13.4	164.0	137.2	26.8
BRINEURA	45.3	38.1	7.2	84.4	77.2	7.2
ALDURAZYME	38.6	40.3	(1.7)	73.8	74.7	(0.9)
KUVAN	28.6	50.6	(22.0)	64.5	101.1	(36.6)
ROCTAVIAN	7.4	—	7.4	8.3	—	8.3
Total net product revenues	$ 702.1	$ 584.7	$ 117.4	$ 1,339.9	$ 1,171.1	$ 168.8

Net Product Revenues include revenues generated from our commercial 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 program, and ALDURAZYME is marketed worldwide by Sanofi. 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.

The increase in Net Product Revenues for the three months ended June 30, 2024 as compared to the three months ended June 30, 2023 was primarily attributed to the following:

- VOXZOGO: higher sales volume from new patients initiating therapy across all regions;
- NAGLAZYME: higher sales volume due to timing of orders in countries that place large government orders, particularly in the Middle East, Europe and Latin America; and
- PALYNZIQ: higher sales volume due to new patients initiating therapy, primarily in the U.S.

These increases were partially offset by the following:

- KUVAN: lower product revenues attributed to increasing generic competition as a result of the loss of market exclusivity.

The increase in Net Product Revenues for the six months ended June 30, 2024 as compared to the six months ended June 30, 2023 was primarily attributed to the following:

- VOXZOGO: higher sales volume from new patients initiating therapy across all regions;

(In millions of U.S. dollars, except as otherwise disclosed)

- PALYNZIQ: higher sales volume from new patients initiating therapy, primarily in the U.S.; and
- NAGLAZYME: higher sales volume due to timing of orders in countries that place large government orders, particularly in the Middle East and Europe.

These increases were partially offset by the following:

- KUVAN: lower product revenues attributed to increasing generic competition as a result of the loss of market exclusivity.

In certain countries, governments place large periodic orders for our products. We expect that the timing of these large government orders will continue to be inconsistent, which has created in the past and may continue to create significant period to period variation in our revenues.

With respect to KUVAN, see also the risk factor "The sale of generic versions of KUVAN by generic manufacturers has adversely affected and will continue to adversely affect our revenues and may cause a decline in KUVAN revenues faster than expected" in "Risk Factors" included in Part II, Item 1A of this Quarterly Report for additional information on risks we face.

Strong demand for VOXZOGO in certain markets had outpaced our projections in recent quarters and we anticipated challenges meeting our estimated VOXZOGO demand during the first half of 2024. We have recently increased fill-finish capacity to meet this demand and continue to implement actions to manage growth and minimize patient impact. While supply constraints have eased in the second quarter of 2024 and we expect to have the ability to meet estimated demand and support ongoing clinical programs, if actual demand continues to exceed estimates, the supply constraint could be prolonged. We do not expect a material impact on our revenues if we successfully execute our manufacturing plans. See "Risk Factors" in Part II, Item 1A of this Quarterly Report for additional information on risk factors that could impact our business and operations.

We face exposure to movements in foreign currency exchange rates, which we expect to continue in future periods. We use foreign currency exchange forward contracts to hedge a percentage of our foreign currency exposure, primarily the Euro. Certain currencies are not included in our hedging program, such as the Argentine Peso. With respect to the risks posed by fluctuations of both hedged and unhedged currencies against the U.S. dollar, see the risk factor "Our international operations pose currency risks, which may adversely affect our operating results and net income" in "Risk Factors" included in Part II, Item 1A of this Quarterly Report for additional information. The following table shows our Net Product Revenues denominated in USD and foreign currencies:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Sales denominated in USD	$ 330.7	$ 275.1	$ 55.6	$ 638.6	$ 572.7	$ 65.9
Sales denominated in foreign currencies	371.4	309.6	61.8	701.3	598.4	102.9
Total net product revenues	$ 702.1	$ 584.7	$ 117.4	$ 1,339.9	$ 1,171.1	$ 168.8

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Unfavorable impact of foreign currency exchange rates on product sales denominated in currencies other than USD	$ (29.9)	$ (21.8)	$ (8.1)	$ (52.6)	$ (50.1)	$ (2.5)

The unfavorable impact for the three and six months ended June 30, 2024 as compared to the three and six months ended June 30, 2023 was primarily driven by weakening of Argentine Peso and Japanese Yen, partially offset by strengthening of the Euro.

(In millions of U.S. dollars, except as otherwise disclosed)

Royalty and Other Revenues

Royalty and Other Revenues include royalties earned on net sales of products sold by third parties, up-front licensing fees and milestones achieved by licensees or sublicensees.

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Royalty and other revenues	$ 9.9	$ 10.6	$ (0.7)	$ 20.9	$ 20.6	$ 0.3

The change in Royalty and Other Revenues for the three and six months ended June 30, 2024 as compared to the three and six months ended June 30, 2023 was relatively flat. We expect to continue to earn royalties from third parties in the future.

Cost of Sales and Gross Margin

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, amortization of technology transfer intangible assets 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, idle plant costs and charges for inventory valuation reserves.

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Total revenues	$ 712.0	$ 595.3	$ 116.7	$ 1,360.9	$ 1,191.7	$ 169.2
Cost of sales	$ 130.5	$ 130.6	$ (0.1)	$ 255.6	$ 266.1	$ (10.5)
Gross margin	81.7 %	78.1 %	3.6 %	81.2 %	77.7 %	3.5 %

Cost of Sales decreased in the three and six months ended June 30, 2024 as compared to the three and six months ended June 30, 2023, primarily from lower per-unit manufacturing costs partially offset by higher sales volumes. Gross margin increased in the current three and six month period due to higher sales volumes for products with higher margin, driven by VOXZOGO sales volume.

We expect gross margin to increase modestly as our product mix is expected to shift, with increasing sales volumes for our higher margin commercial products.

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 commercial 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, R&D facilities and regulatory costs.

(In millions of U.S. dollars, except as otherwise disclosed)

We group all of our R&D activities and related expense into three categories: (i) research and early pipeline, (ii) later-stage clinical programs and (iii) marketed products as follows:

Category	Description
Research and early pipeline	R&D expense incurred in activities substantially in support of early research through the completion of phase 2 clinical trials, including drug discovery, toxicology, pharmacokinetics and drug metabolism and process development.
Later-stage clinical programs	R&D expense incurred in or related to phase 3 clinical programs intended to result in registration of a new product or a new indication for an existing product primarily in the U.S. or the EU.
Marketed products	R&D expense incurred in support of our marketed products that are authorized to be sold primarily in the U.S. or the EU. Includes clinical trials designed to gather information on product safety (certain of which may be required by regulatory authorities) and their product characteristics after regulatory approval has been obtained, as well as the costs of obtaining regulatory approval of a product in a new market after approval in either the U.S. or EU has been obtained.

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.

R&D consisted of the following:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Research and early pipeline	$ 106.1	$ 90.3	$ 15.8	$ 226.5	$ 176.5	$ 50.0
Later-stage clinical programs	7.7	—	7.7	7.7	30.7	(23.0)
Marketed Products	70.0	87.1	(17.1)	154.6	142.0	12.6
Total R&D	$ 183.8	$ 177.4	$ 6.4	$ 388.8	$ 349.2	$ 39.6

The increase in R&D expense for the three months ended June 30, 2024 as compared to the three months ended June 30, 2023 was primarily due to the following:

- Research and early pipeline: higher spend related to pre-clinical activities for new VOXZOGO indications for idiopathic short stature (ISS) and multiple genetic short stature pathway conditions (PC) and our prioritized pipeline comprised of BMN 333, BMN 349 and BMN 351; and
- Later-stage clinical programs: higher spend on clinical activities related to VOXZOGO for the treatment of hypochondroplasia.

These increases were partially offset by the following:

- Marketed products: lower spend on ROCTAVIAN and VOXZOGO for achondroplasia as we shift resources to the new VOXZOGO indications and our prioritized pipeline.

The increase in R&D expense for the six months ended June 30, 2024 as compared to the six months ended June 30, 2023 was primarily due to the following:

- Research and early pipeline: higher spend related to pre-clinical activities for new VOXZOGO indications for ISS and PC and our prioritized pipeline; and
- Marketed products: higher spend primarily related to ROCTAVIAN, which was moved to Marketed products following Food and Drug Administration approval in the second quarter of 2023.

These increases were partially offset by the following:

- Later-stage clinical programs: lower spend on ROCTAVIAN, which was moved to Marketed products following Food and Drug Administration approval in the second quarter of 2023, partially offset by higher spend on the new VOXZOGO indication for hypochondroplasia.

(In millions of U.S. dollars, except as otherwise disclosed)

We expect R&D expense to increase in future periods, primarily due to increased spending for the new VOXZOGO indications and research and early pipeline programs.

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.

SG&A consisted of the following:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
S&M	$ 122.8	$ 115.6	$ 7.2	$ 242.2	$ 233.3	$ 8.9
G&A	140.2	90.5	49.7	246.7	183.8	62.9
Total SG&A	$ 263.0	$ 206.1	$ 56.9	$ 488.9	$ 417.1	$ 71.8

S&M consisted of the following:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Enzyme Products	$ 56.4	$ 53.7	$ 2.7	$ 111.6	$ 112.7	$ (1.1)
VOXZOGO	31.7	26.3	5.4	62.4	51.0	11.4
ROCTAVIAN	24.1	23.9	0.2	48.0	47.4	0.6
Other	10.6	11.7	(1.1)	20.2	22.2	(2.0)
Total S&M	$ 122.8	$ 115.6	$ 7.2	$ 242.2	$ 233.3	$ 8.9

The increase in S&M expense for the three months ended June 30, 2024 as compared to the three months ended June 30, 2023 was primarily attributed to continued global expansion of VOXZOGO for achondroplasia and higher partner distribution fees. The increase in S&M expense for the six months ended June 30, 2024 as compared to the six months ended June 30, 2023 was primarily a result of the continued global expansion of VOXZOGO for achondroplasia.

The increase in G&A expense for the three and six months ended June 30, 2024 as compared to the three and six months ended June 30, 2023 was primarily due to severance and restructuring costs associated with our portfolio strategy review and the associated organizational redesign efforts announced in the second quarter of 2024. The increase in G&A expense for the six months ended June 30, 2024 as compared to the six months ended June 30, 2023 also included incremental administrative costs, including consulting and legal fees.

We expect SG&A expense to increase in future periods as a result of ongoing corporate initiatives and the continued market expansion of our commercial products.

(In millions of U.S. dollars, except as otherwise disclosed)

Intangible Asset Amortization and Gain on Sale of Nonfinancial Assets

Intangible Asset Amortization and Gain on Sale of Nonfinancial Assets were as follows:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Amortization of intangible assets	$ 14.3	$ 15.6	$ (1.3)	$ 28.6	$ 31.3	$ (2.7)
Gain on sale of nonfinancial assets	$ —	$ —	$ —	$ 10.0	$ —	$ 10.0

Amortization of intangible assets – the decrease in expense for the three and six months ended June 30, 2024 as compared to the three and six months ended June 30, 2023 was due to an intangible asset becoming fully amortized during the fourth quarter of 2023.

Gain on Sale of Nonfinancial Assets – in the first quarter of 2024, we recognized a gain of $10.0 million due to a third party's achievement of a regulatory approval milestone related to previously sold intangible assets.

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.

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Interest income	$ 19.8	$ 12.6	$ 7.2	$ 39.2	$ 24.6	$ 14.6

The increase in Interest Income for the three and six months ended June 30, 2024 compared to the three and six months ended June 30, 2023 was primarily due to higher balances and higher yields on our cash equivalents and investment portfolio. We expect Interest Income to increase moderately over the next 12 months due to anticipated higher overall returns on our cash equivalents and investments.

Interest Expense

We incur interest expense primarily on our convertible debt. Interest Expense for the periods presented was as follows:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Interest expense	$ 3.6	$ 3.8	$ (0.2)	$ 7.1	$ 7.5	$ (0.4)

Interest Expense for the three and six months ended June 30, 2024 as compared to the three and six months ended June 30, 2023 was relatively flat. We expect Interest Expense to decrease over the next 12 months due to the settlement of our convertible debt that matured on August 1, 2024. See Note 6 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our debt.

Other Expense, Net

Other Expense, Net for the periods presented was as follows:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Other expense, net	$ 4.5	$ 3.6	$ 0.9	$ 3.3	$ 17.5	$ (14.2)

(In millions of U.S. dollars, except as otherwise disclosed)

The increase in Other Expense, Net for the three months ended June 30, 2024 compared to the three months ended June 30, 2023 was primarily due to a loss on non-marketable securities partially offset by lower foreign currency transaction losses in the comparable period in 2023. The decrease in Other Expense, Net for the six months ended June 30, 2024 compared to the six months ended June 30, 2023 was primarily due to the decrease in loss on non-marketable securities partially offset by lower foreign currency transaction losses.

Provision for Income Taxes

The Provision for Income Taxes for the periods presented was as follows:

	Three Months Ended June 30,			Six Months Ended June 30,		
	2024	2023	Change	2024	2023	Change
Provision for income taxes	$ 25.0	$ 14.8	$ 10.2	$ 41.8	$ 20.7	$ 21.1

The increase in Provision for Income Taxes for the three and six months ended June 30, 2024 as compared to the three and six months ended June 30, 2023 was primarily due to higher pre-tax income and a lower tax benefit related to stock option exercises.

There was no material impact resulting from the adoption of Pillar Two of the Organisation for Economic Co-operation and Development (OECD) Base Erosion and Profit Shifting Project on our financial statements.

Financial Condition, Liquidity and Capital Resources

Our cash, cash equivalents, and investments as of June 30, 2024 and December 31, 2023 were as follows:

	June 30, 2024	December 31, 2023	Change
Cash and cash equivalents	$ 972.1	$ 755.1	$ 217.0
Short-term investments	252.2	318.7	(66.5)
Long-term investments	557.1	611.1	(54.0)
Cash, cash equivalents and investments	$ 1,781.4	$ 1,684.9	$ 96.5

We believe cash generated from sales of our commercial products, in addition to our cash, cash equivalents and short-term investments will be sufficient to satisfy our liquidity requirements for at least the next 12 months. We believe we will meet longer-term expected future cash requirements and obligations through a combination of cash flows from operating activities and available cash and long-term investment balances. We will need to raise additional funds by issuing equity, debt or convertible securities, taking loans or entering into collaborative or other agreements if we are unable to satisfy our liquidity requirements. For example, we may require additional financing to fund the repayment of our convertible debt due in 2027, 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. The timing and mix of our funding alternatives 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 settle our convertible debt in cash.

We are mindful that conditions in the current macroeconomic environment, such as inflation, changes in interest and foreign currency exchange rates, natural disasters, geopolitical instability, and supply chain disruptions could affect our ability to achieve our goals. In addition, we sell our products in certain 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

(In millions of U.S. dollars, except as otherwise disclosed)

monitor these conditions and will attempt to adjust our business processes, as appropriate, to mitigate macroeconomic risks to our business.

Our cash flows are summarized as follows:

	Six Months Ended June 30,		
	2024	2023	Change
Net cash provided by (used in) operating activities	$ 165.7	$ (3.9)	$ 169.6
Net cash provided by (used in) investing activities	$ 76.2	$ (0.5)	$ 76.7
Net cash used in financing activities	$ (30.2)	$ (28.8)	$ (1.4)

The increase in net cash provided by operating activities in the six months ended June 30, 2024 compared to June 30, 2023 was primarily attributed to the improved operating performance and timing of cash receipts from our customers, partially offset by the timing of payments to vendors and payments of income taxes.

The increase in net cash provided by investing activities in the six months ended June 30, 2024 compared to June 30, 2023 was primarily attributable to lower purchases of available-for-sale debt securities net of maturities and a $10.0 million milestone received in connection with the sale of previously sold intangible assets, partially offset by an increase in purchases of intangible assets.

The increase in net cash used in financing activities in the six months ended June 30, 2024 compared to June 30, 2023 was primarily due to lower proceeds from stock option exercises, partially offset by the absence of milestone payments to third parties.

Financing

Our $1.1 billion (undiscounted) of total convertible debt as of June 30, 2024 will impact our liquidity due to the semi-annual cash interest payments as well as the repayment of the principal amount, if not converted. As of June 30, 2024, our indebtedness consisted of our 0.599% senior subordinated convertible notes due in 2024 and our 1.25% senior subordinated convertible notes due in 2027, which, if not converted, will be required to be repaid in cash at maturity in August 2024 and May 2027, respectively. As of June 30, 2024, the outstanding principal of our convertible notes due in 2024 (2024 Notes) was classified as a current liability.

Our 2024 Notes matured on August 1, 2024, subsequent to quarter-end. Substantially all holders of the 2024 Notes were repaid with cash, totaling approximately $495.0 million in cash. No gain or loss was incurred upon the extinguishment.

For additional information related to our convertible debt, see Note 6 to our accompanying Condensed Consolidated Financial Statements and Note 10 - *Debt* to the Consolidated Financial Statements accompanying our Annual Report on Form 10-K for the year ended December 31, 2023.

Material Cash Requirements

Purchase Obligations

As of June 30, 2024, we had obligations of approximately $473.0 million, of which $291.1 million is expected to be paid in 2024. Our purchase obligations are primarily related to firm purchase commitments entered into in the normal course of business to procure active pharmaceutical ingredients, certain inventory-related items, certain third-party R&D services, production services and facility construction services. The amount also includes hosting fees and other enterprise resource planning (ERP) system implementation costs for which we are committed.

Other Obligations

As of June 30, 2024, we were subject to contingent payments considered reasonably possible of $495.6 million, of this amount we may pay up to $10.9 million over the next 12 months if certain contingencies are met. See Note 12 to our accompanying Condensed Consolidated Financial Statements for additional discussion on our contingent obligations.

Our lease and unrecognized tax benefits as of June 30, 2024 have not materially changed from those discussed in "Financial Condition, Liquidity and Capital Resources" in Part II, Item 7 of our Annual Report on Form 10-K for the year ended December 31, 2023.

See Note 12 to our accompanying Condensed Consolidated Financial Statements for additional information on our commitments.

Critical Accounting Estimates

In preparing our Condensed Consolidated Financial Statements in accordance with 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, revenues 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. Historically, our assumptions, judgments and estimates relative to our critical accounting estimates have not differed materially from actual results.

There have been no significant changes to our critical accounting estimates during the six months ended June 30, 2024, compared to those 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, 2023, filed with the SEC on February 26, 2024.

Recent Accounting Pronouncements

See Note 1 to our accompanying Condensed Consolidated Financial Statements for a description of recent accounting pronouncements, if any, and our expectation of their impact on our results of operations and financial condition.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Our market risks during the six months ended June 30, 2024 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, 2023.

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, at the reasonable assurance level, as of June 30, 2024.

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) Changes 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 continue to utilize the Committee of Sponsoring Organizations of the Treadway Commission (COSO) 2013 Framework on internal control. We rely extensively on information systems and technology to manage our business, including integrated supply chain operations, and global consolidated financial results. We are currently implementing a new global enterprise resource planning (ERP) system, which will replace existing operating and financial systems. The ERP system is designed to accurately maintain our financial records, support integrated supply chain and other operational functionality, and provide timely information to our management team related to the operation of the business. We are implementing the ERP system in phases through 2025, with post-implementation activities following thereafter. As the implementation and post-implementation activities take place, we will have changes to certain of our processes and procedures, and we will evaluate quarterly whether the changes materially affect our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

On January 19, 2023 and May 30, 2023, certain of our officers and directors were named as defendants in two shareholder derivative actions filed in the Delaware Court of Chancery. The complaints assert, inter alia, breach of fiduciary duty claims arising from the facts underlying a securities class action related to ROCTAVIAN that was previously settled in the United States District Court for the Northern District of California. The derivative complaints seek unspecified monetary damages, internal governance reforms by the Company, attorneys' fees and costs, and any other relief the court may deem just and proper. The parties in the derivative lawsuits have entered into a stipulation of settlement, that, subject to final approval by the Court of Chancery, will resolve the derivative lawsuits. The Court of Chancery held a final approval hearing on July 23, 2024 and did not approve the settlement. Instead, it instructed the plaintiffs to voluntarily dismiss the case or brief a motion to dismiss.

On April 4, 2024, a purported stockholder class action was filed against us and our Board of Directors in the Delaware Court of Chancery. The complaint also named as defendants Elliott Investment Management L.P., Elliott Associates, L.P., and Elliott International, L.P., which are parties to the Cooperation Agreement with the Company. The complaint asserted a claim for declaratory judgment, seeking an order that certain provisions of the Cooperation Agreement are invalid, and, on April 4, 2024, the plaintiff moved for expedited proceedings as to the claim for declaratory judgment. In addition, the complaint asserted a claim for breach of fiduciary duty against certain directors in connection with approval of the Cooperation Agreement, as well as a claim against the Elliott parties for aiding and abetting the directors' alleged breaches of fiduciary duty. On April 11, 2024, the Elliott parties executed a waiver of the challenged provisions in the Cooperation Agreement. In light of that waiver, on April 16, 2024, the plaintiff filed a stipulation and proposed order to dismiss the action as moot, with the court to retain jurisdiction to determine plaintiff's counsel's application for an award of attorneys' fees and expenses. Also on April 16, 2024, the Court granted the order, dismissing all claims with prejudice, as to the named plaintiff only. The Company subsequently agreed to pay the attorneys' fees and expenses of the plaintiff in full satisfaction of any and all claims by plaintiff and all of his counsel for fees and expenses in the action. On August 1, 2024, the Court entered an order closing the action, subject to the Company filing and affidavit with the Court regarding issuance of notice.

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, 2023, which was filed with the SEC on February 26, 2024.

Business and Operational Risks

If we fail to obtain and maintain 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. For all our products except ROCTAVIAN, 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 revenues and gross margin 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. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future based on new legislation, the availability of alternative therapies and their pricing, coverage and reimbursement decisions by third-party payers, or other factors. 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 European Union (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 pricing and/or 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 margin may be adversely affected.

As compared to our other, more traditional products, gene therapy products may present additional challenges with respect to the pricing, coverage, reimbursement, and acceptance of the product.

In addition to the risks set forth in this Risk Factors section associated with commercializing more traditional pharmaceutical drugs, there are additional, unique commercial risks associated with gene therapy products like ROCTAVIAN. Due to the relative novelty of gene therapy and the potential to provide extended duration therapeutic treatment with a one-time administration, we face uncertainty with respect to the pricing, coverage and reimbursement of these products. In order to recover our research and development costs and commercialize one-time treatments on a profitable basis, the cost of a single administration of ROCTAVIAN is substantial, and it is likely other gene therapy products would also require relatively high prices. Therefore, coverage and reimbursement by governments and other third-party payers is essential for the vast majority of patients to be able to afford ROCTAVIAN or other gene therapy products that we may commercialize in the future. Accordingly, sales of our gene therapy products will depend substantially 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 our gene therapy products.

With respect to ROCTAVIAN specifically, we have entered into, and plan to enter into additional, outcomes-based agreements for the product with third-party payers to assist with realizing the value and sharing the risk of a one-time treatment, which make us subject to potential repayments if a patient does not respond to therapy or the therapeutic effect of the drug falls below specified thresholds. Although we will record reserves for potential refunds under the outcomes-based agreements for ROCTAVIAN in the same period as sales, our revenues and financial results could be adversely affected if our assumptions underlying our refund reserves differ from actual experience or otherwise underestimate refund obligations. Additionally, the novelty and increased complexity of reimbursement with outcomes-based arrangements heightens the risk that our price reporting may be inaccurate or delayed, which may result in fines and liability.

We also face uncertainty as to whether gene therapy will gain the acceptance of the public or the medical community. The commercial success of ROCTAVIAN or any other gene therapy product candidate that may be approved in the future will depend, in part, on the acceptance of physicians, patients and third-party payers of gene therapy products in general, and our product in particular, as medically necessary, cost-effective and safe. In particular, our success will depend upon physicians prescribing our product in lieu of existing treatments they are already familiar with and for which greater clinical data may be available. Moreover, physicians and patients may delay acceptance of one of our gene therapy treatments until the product has been on the market for a certain amount of time. Although administration of a gene therapy product like ROCTAVIAN is intended to correct an inborn genetic defect for at least several years, there is a risk that the therapeutic effect will not be durable and production of the desired protein or ribonucleic acid will decrease more quickly or cease entirely earlier than expected. If the therapeutic effect decreases significantly or ceases entirely, it is uncertain whether redosing is possible or would be effective. Furthermore, because gene therapy 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. Additionally, 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. 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 successful commercialization of, and demand for, ROCTAVIAN or future gene therapy products.

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

All of our products target diseases with relatively 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 and maintain 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 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 revenues 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 for seven years, along with other regulatory exclusivities that could block approval) or commercialize their products before we do. With respect to ROCTAVIAN, we face a highly developed and competitive market for hemophilia A treatments. As we commercialize ROCTAVIAN, we may face intense competition from large pharmaceutical companies with extensive resources and established relationships in the hemophilia A community. If we do not compete successfully, our revenues 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.

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. 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 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 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 like gene therapy products, 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, 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 target diseases that we are also targeting or may target in the future, 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.

The sale of generic versions of KUVAN by generic manufacturers has adversely affected and will continue to adversely affect our revenues and may cause a decline in KUVAN revenues faster than expected.

Generic versions of KUVAN are available in several countries around the world, including multiple generic versions in the U.S. and the EU. This generic competition has adversely affected and will continue to adversely affect our revenues from KUVAN, and we cannot accurately predict the rate of decline of KUVAN revenues in these countries. We are also aware that manufacturers are challenging our patent portfolio related to KUVAN in several jurisdictions, and several generic versions of KUVAN have been approved either centrally by the European Commission (EC) or on a country-by-country basis throughout the EU. If these patent challenges are successful, or if a manufacturer chooses to offer a generic version of KUVAN, notwithstanding our existing patents, our revenues from KUVAN may decline faster than expected.

If we do not achieve our projected development goals in the timeframes we announce or fail to achieve such goals, the commercialization of our product candidates may be delayed or never occur 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 or the milestones may never be achieved, in many cases for reasons beyond our control. For example, in 2021 and early 2022, we announced that we planned to resubmit our Biologics License Application (BLA) for ROCTAVIAN to the Food and Drug Administration (FDA) in the first half of 2022; however, we did not file the BLA until the third quarter of 2022 due to the additional time we needed to include supplemental information and analyses of data requested by the FDA. If we do not meet development milestones as publicly announced, the commercialization of our products may be delayed or never occur and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

We have in the past entered and may in the future enter into licensing arrangements, and we may not realize the benefits of such licensing arrangements.

We have in the past entered and may in the future enter into licensing arrangements with third parties. It is possible that we may not achieve financial or strategic benefits that justify a specific license, or we may otherwise not realize the benefits of such licensing arrangement. Further, licensing arrangements impose various diligence, milestone and royalty payment and other obligations on us. If we fail to comply with our obligations under any current or future licenses, our licensors may have the right to terminate these license agreements, which could harm our business prospects, financial condition and results of operations. Additionally, counterparties to our license agreements have in the past alleged and may in the future allege that we have breached a license agreement, which can result in litigation or other disputes that can divert management's attention away from our business and require us to expend resources, as well as potentially having to negotiate new or reinstated licenses with less favorable terms. Any such situation could adversely affect our business, financial condition, and results of operations.

Activist investor actions threatened or commenced against us have and could in the future cause us to incur substantial costs, divert management's attention and resources, cause uncertainty about the strategic direction of our business and adversely affect our business, financial position and results of operations.

We have been, and may in the future be, subject to activities initiated by activist investors. In December 2023, we entered into a Cooperation Agreement with Elliott Investment Management L.P., Elliott Associates, L.P. and Elliott International, L.P. (collectively, Elliott). We may not be successful in engaging constructively with one or more investors in the future despite our efforts to maintain constructive and ongoing communications with all investors, including Elliott. Resulting actions taken by activist investors from time to time have and could in the future conflict with our strategic direction, divert the attention of our Board of Directors, management, and employees, be costly and time-consuming, and disrupt the momentum in our business and operations, as well as our ability to execute our strategic plan. These types of actions may also create perceived uncertainties as to the future direction of our business or strategy, which may be exploited by our competitors and may make it more difficult to attract and retain qualified personnel, and may impact our relationships with investors, vendors, customers and other third parties. These types of actions could also impact the market price and the volatility of our common stock. In addition, we may choose to initiate, or may become subject to, litigation as a result of activist investor actions, which would serve as a further distraction to our Board of Directors, senior management and employees and could require us to incur significant additional costs.

Regulatory Risks

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 revenues 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 regulatory approval to market and sell our product candidates. For example, in the U.S., we must obtain FDA approval for each product candidate that we intend to commercialize, and in the EU, we must obtain approval from the EC, based on the opinion of the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA). The FDA and EC approval processes are typically lengthy and expensive, and approval is never certain. To obtain regulatory approval, we must first show that our product candidates are safe and effective for target indications through preclinical studies and clinical trials. Preclinical studies and clinical development are long, expensive and uncertain processes. Completion of clinical trials may take several years, and failure may occur at any stage of development. The length of time required varies substantially according to the type, complexity, novelty and intended use of a product candidate. Interim results of a preclinical test or clinical trial do not necessarily predict final results, and acceptable results in early clinical trials may not be repeated in later clinical trials. 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 authority will mean that other authorities will also approve the same product candidate. Similarly, in the EU, a positive CHMP opinion for approval of a product candidate does not guarantee that the EC will approve the product candidate. Moreover, regulatory authorities may approve a product candidate for fewer or more limited indications than requested. 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, the EC and the 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 EC approval. Moreover, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Approval by the FDA or EC does not ensure approval by regulatory authorities in other countries, and approval by one or more non-U.S. regulatory authorities does not ensure approval by regulatory authorities in other non-U.S. countries or by the FDA or EC. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. The non-U.S. regulatory approval process may include all of the risks associated with obtaining FDA or EC approval. We may not obtain non-U.S. 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.

We also rely on independent third-party Contract Research Organizations (CROs) to file some of our non-U.S. marketing applications, and while we keep a close oversight on the activities we delegate to CROs, 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.

Although the FDA, the EC and the EMA have programs to facilitate expedited development and accelerated approval processes, the timelines agreed under legislative goals or mandated by regulations are subject to the possibility of substantial delays. Accordingly, even if any of our applications receives a designation to facilitate expedited development and accelerated approval processes, these designations may not result in faster review or approval for our product candidates compared to product candidates considered for approval under conventional procedures and, in any event, do not assure ultimate approval of our product candidates by regulatory authorities. In addition, the FDA, the EC, the EMA and other comparable international regulatory authorities have substantial discretion over the approval process for pharmaceutical products. These regulatory authorities may not agree that we have demonstrated the requisite level of product safety and efficacy to warrant approval and may require, and in the past have required, additional data. If we fail to obtain regulatory approval for our product candidates, we will be unable to market and sell those product candidates, which would have a negative effect on our business and financial condition.

Regulatory authorities 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 product candidates or lead to significant post-approval studies, limitations or restrictions. For example, on August 18, 2020, the FDA issued a Complete Response Letter (CRL) to our BLA for ROCTAVIAN for the treatment of adults with severe hemophilia A. In the CRL, the FDA introduced a new request for two-year follow-up safety and efficacy data on all study participants from our ongoing Phase 3 study of ROCTAVIAN. In January 2022, we announced results from the requested two-year data analysis from our Phase 3 study. In the third quarter of 2022, we resubmitted our BLA, and the FDA subsequently accepted our submission with an original Prescription Drug User Fee Act (PDUFA) target action date of March 31, 2023. In early 2023, we supplemented our BLA by submitting our three-year analysis of the global Phase 3 study of ROCTAVIAN, which the FDA deemed to be a Major Amendment to our BLA due to the substantial amount of additional data, and extended the PDUFA target action date by three months. The FDA approved ROCTAVIAN for the treatment of adults with severe hemophilia A on June 29, 2023. Further, on April 26, 2023, the EC adopted a proposal for a new Directive and Regulation to

revise the existing pharmaceutical legislation. If adopted in the form proposed, the recent EC proposals to revise the existing EU laws governing authorization of medicinal products may result in a decrease in data and market exclusivity for our product candidates in the EU.

In addition, some of our product candidates are intended to be used in combination with a medical device, such as an injector or other delivery system. Some of these products intended to be used with a medical device may be regulated as "combination products" in the U.S. and the EU, which are generally defined as products consisting of components from two or more regulatory categories (e.g., drug/device, device/biologic, drug/biologic). In the U.S., 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. In the EU, medical devices and medicinal products are regulated separately, through different legislative instruments. The related applicable requirements will vary depending on the type of drug-device combination product. If, for example, a device intended to administer a medicinal product is sold together with such medicinal product in such a way that they form a single integral product which is intended exclusively for use in the given combination and which is not reusable, that single integral product is regulated as a medicinal product. In addition, the relevant general safety and performance requirements (GSPRs) established for medical devices by EU medical devices legislation apply to the device component of such combination products. In addition, some of our products require use with an *in vitro* companion diagnostic. For example, ROCTAVIAN is approved with a companion diagnostic test intended to detect pre-existing anti-AAV5 antibodies, which may render the gene therapy less effective or ineffective. Our other products and product candidates may also require use with an *in vitro* companion diagnostic if the FDA determines that the companion diagnostic is essential for safe and effective use of the product candidate. The FDA generally will require approval or clearance of the diagnostic, known as a companion diagnostic, at the same time that the FDA approves the therapeutic product. Most companion diagnostics require approval of a premarket approval application. In the EU, companion diagnostics are deemed to be *in vitro* diagnostic medical devices and must conform with the applicable GSPRs. To demonstrate compliance with the GSPRs, companion diagnostics must undergo a conformity assessment by a Notified Body. If the related medicinal product has been, or is in the process of being, authorized through the centralized procedure for the authorization of medicinal products, the Notified Body will, before it can issue a CE Certificate of Conformity, be required to seek a scientific opinion from the EMA on the suitability of the companion diagnostic for use in relation to the medicinal product concerned. For medicinal products that have been or are in the process of authorization through any other route provided in EU legislation, the Notified Body must seek the opinion of the national competent authority of an EU Member State. Our product candidates intended for use with separately regulated devices, such as companion diagnostics, 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, clearances, or certifications. Where approval of the drug or biologic product and device is sought under a single application, such as a drug with an injector or delivery system, the increased complexity of the review process may delay approval. The FDA and EU review processes and related criteria are complex, which could also lead to delays in the approval process. In addition, because these 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.

Furthermore, despite our recent success obtaining regulatory approval for ROCTAVIAN in the U.S. and conditional approval in the EU, we may experience regulatory challenges for other gene therapy product candidates 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, the FDA and EC have only approved a relatively small number of vector-based gene therapy products thus far. 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 future gene therapy product candidates in any jurisdiction. Regulatory requirements governing gene and cell therapy products are still evolving and may continue to change in the future. Further, the FDA continues to develop and publish new guidance and policies, generally, by releasing one or more gene therapy-specific guidance documents each year. These guidance documents and other recent policy statements demonstrate that regulatory requirements for gene therapies are likely to continue to evolve based upon factors such as the intended disease or class of diseases, product type or mechanism of action, broader considerations such as the kinds of evidence that will be required for gene therapy products to take advantage of expedited development programs, and the experiences obtained by FDA when applying their legal and regulatory authorities to an evolving field, like gene therapy products. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring our gene therapy product candidates to market could have a negative effect on our business and financial condition.

From time to time during the development and regulatory approval process for our products and product candidates, we engage in discussions with the FDA, the EC, the EMA and other 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 authorities 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. Moreover, sometimes different regulatory authorities 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, the EC, the EMA and other comparable international 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 EC, 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 revenues 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.

Our products have received regulatory approval to be commercially marketed and sold in the U.S., the EU, and certain other countries except ROCTAVIAN, which has received regulatory approval to be commercially marketed in the U.S. and conditional 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 EC, the EMA and/or other comparable international and national 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.

In addition, in the EU, the marketing authorization for BRINEURA was granted under “exceptional circumstances”. As a result, the risk-benefit balance of BRINEURA is reviewed annually and the marketing authorization may be withdrawn if the risk-benefit ratio is no longer favorable. The conditional marketing authorization for ROCTAVIAN is, moreover, valid for one year and must be reviewed annually until all related conditions have been fulfilled to permit transfer to a full authorization. Failure to continue to show favorable risk-benefit balance for BRINEURA or satisfy the conditions related to ROCTAVIAN’s conditional marketing authorization could result in the withdrawal of the marketing approvals for these products.

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 and Summary of Product Characteristics. In particular, a product may not be promoted for uses that are not approved by the FDA or the EC as reflected in the product’s approved labeling. Although the FDA and other comparable international and national regulatory authorities do not regulate a physician’s choice of drug treatment made in the physician’s independent medical judgment, they do restrict promotional communications from companies or their sales force with respect to off-label uses of products for which marketing clearance has not been issued. The FDA and other national competent authorities or international regulatory authorities 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 are not able

to promote any products we develop for indications or uses for which they are not approved. Additionally, in the EU, it is prohibited to promote prescription drugs to the general public and we are therefore limited to promote our products exclusively to healthcare professionals. Public prosecutors, industry associations, healthcare professionals and other members of the public closely scrutinize advertising and promotion of any product in the EU.

Moreover, if original FDA approval for one of our product candidates is granted via the accelerated approval pathway, we will 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 with due diligence could result in the withdrawal of the FDA's marketing approval for a product candidate. For example, VOXZOGO is approved in the U.S. under accelerated approval based on an improvement in annualized growth velocity. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory studies. To fulfill this post-marketing requirement, we intend to use our ongoing open-label extension studies compared to available natural history. In addition, the FDA and the EC often require post-marketing testing and surveillance to monitor the effects of products. The FDA, the EC and other comparable international regulatory authorities 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:

- the issuance of safety alerts, press releases or other communications containing warnings about related products;
- modifications to promotional materials or corrective information to healthcare professionals;
- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- suspensions or restrictions on our operations, including 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;
- suspended or withdrawn regulatory approvals;
- refusal or delays 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 EC or any other comparable international regulatory authorities withdraws its approval of a product, we will be unable to generate revenues 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.

To obtain regulatory approval to market our products, preclinical studies and costly and lengthy clinical trials are required and the results of the studies and trials are highly uncertain. Likewise, preliminary, initial or interim data from clinical trials should be considered carefully and with caution because the final data may be materially different from the preliminary, initial or interim data, particularly as more patient data become available.

As part of the drug development process, we must conduct, at our own expense, preclinical studies in the laboratory, including studies in animals, and clinical trials on humans for each product candidate. The number of preclinical studies and clinical trials that regulatory authorities require varies depending on the product candidate, the disease or condition the drug is being developed to address and regulations applicable to the particular drug. Generally, new drugs for diseases or conditions that affect larger patient populations, are less severe, or are treatable by alternative strategies must be validated through additional preclinical and clinical trials and/or clinical trials with higher enrollments. With respect to our early-stage product candidates, we may need to

perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays to our development timeline. Furthermore, even if we obtain favorable results in preclinical studies, the results in humans may be significantly different. After we have conducted preclinical studies, we must demonstrate that our product candidates are safe and efficacious for the intended indication and for use in the targeted human patients in order to receive regulatory approval for commercial sale. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, and favorable data from interim analyses do not ensure the final results of a trial will be favorable. From time to time, we have published and may in the future publish or report preliminary, initial or interim data from our clinical trials. Preliminary, initial or interim data from our clinical trials may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. In this regard, such data may show initial evidence of clinical benefit, but as patients continue to be followed and more patient data become available, there is a risk that any therapeutic effects will not be durable in patients and/or will decrease over time or cease entirely. Preliminary, initial or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from such preliminary, initial or interim data. As a result, preliminary, initial or interim data should be considered carefully and with caution until the final data are available.

Product candidates may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials, or despite having favorable data in connection with an interim analysis. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Also, as noted above, we do not always follow the advice of regulatory authorities or comply with all of their requests regarding the design of our clinical programs. In those cases, we may choose a development program that is inconsistent with the advice of regulatory authorities, which may limit the jurisdictions where we conduct clinical trials and/or adversely affect our ability to obtain approval in those jurisdictions where we do not follow the regulatory advice.

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

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

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 revenues 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 been under scrutiny for many years 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. For example, in the EU, governments influence the price of medicinal products through their pricing and reimbursement rules and control of national healthcare systems that fund a large part of the cost of those products to consumers. EU Member States are free to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed to by the government. An EU Member State may approve a specific price for the medicinal product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market, including volume-based arrangements, caps and reference pricing mechanisms. Other EU Member States allow companies to fix their own prices for medicines but monitor and control company profits. The downward pressure on healthcare costs in general, particularly prescription medicines, has become very intense. Pharmaceutical products may face competition from lower-priced products in foreign countries that have placed price controls on pharmaceutical products and may also compete with imported foreign products. Furthermore, there is no assurance that a product will be considered medically reasonable and necessary for a specific indication or cost-effective by third-party payers. There is also no assurance that an adequate level of reimbursement will be established even if coverage is available or that the third-party payers' reimbursement policies will not adversely affect our business.

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 revenues and results of operations.

Government healthcare reform could increase our costs and adversely affect our revenues 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., there have been and continue to be a number of legislative initiatives to contain healthcare costs. In the U.S., there have been several recent congressional inquiries, proposed and enacted federal and state legislation and executive action 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. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. Recently, several healthcare reform initiatives culminated in the enactment of the Inflation Reduction Act (IRA) in August 2022, which allows, among other things, U.S. Department of Health and Human Services (HHS) to negotiate the selling price of a statutorily specified number of drugs and biologics each year that the CMS reimburses under Medicare Part B and Part D. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for biologics) can be selected by CMS for negotiation. The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. HHS has and will continue to issue and update guidance as these programs are implemented. Manufacturers that fail to comply with the IRA may be subject to various penalties, including civil monetary penalties. The IRA's provisions are taking effect progressively starting in 2023, although they may be subject to legal challenges. Thus, while it is unclear how the IRA will be implemented, it will likely have a significant impact on the pharmaceutical industry.

Prior to the IRA, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, 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 executive, judicial and congressional challenges to certain aspects of the PPACA. Although the PPACA has generally been upheld thus far, it is unclear how continued challenges to the law may 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.

In addition, individual states in the U.S. have also increasingly enacted laws and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, price disclosure and reporting requirements, 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 Member States, 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 commercial products and any 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 Member States and other non-U.S. 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. If the price of one of our products decreases substantially in a reference price country, it could impact the price for that product in other countries. Consequently, a downward trend in prices of our products in some countries could contribute to similar downward trends elsewhere, which would have a material adverse effect on our revenues and results of operations. Moreover, some EU Member States require the completion of additional studies that compare the cost-effectiveness of a particular medicinal product candidate to currently available therapies. This Health Technology Assessment (HTA) process, which is currently governed by the national laws of the individual EU Member States, is the procedure according to which the assessment of the public health impact, therapeutic impact and the economic and societal impact of use of a given medicinal product in the national healthcare systems of the individual country is conducted. The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. In 2022, the EC adopted the HTA regulation, which is intended to boost cooperation among EU Member States in evaluating new medicinal products. The HTA regulation will apply starting in 2025 and may result in increased downward pricing pressure in the EU.

We anticipate that the IRA, PPACA and 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. 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.

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. or as a condition that affects more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the costs of development of said drug will be recovered from sales in the U.S. In the EU, pursuant to the Regulation (EC) No. 141/2000 (the Orphan Regulation), as implemented by Regulation (EC) No. 847/2000, orphan drug designation is available if a sponsor can establish that: (1) the medicine is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than five in 10,000 people in the EU at the time the application is made, or, (2) that it is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives derived from the orphan status, it is unlikely that the marketing of the medicine in the EU would generate sufficient return to justify the necessary investment. In both cases, 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 medicine will be of significant benefit to those affected by that condition.

In the U.S., the company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the designated 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 for the approved therapeutic indication (extendable to twelve years for orphan drugs that have complied with an agreed Pediatric Investigation Plan (PIP) pursuant to Regulation 1901/2006), during which the EMA cannot accept another marketing authorization application or accept an application to extend existing authorizations for similar medicinal products for the same indication and no related marketing authorization (MA) can be granted. MAs may also be granted to a similar medicinal product with the same orphan indication if: (i) the applicant can establish that the second medicinal product, although similar to the orphan medicinal product already authorized is safer, more effective or otherwise clinically superior to the orphan medicinal product already authorized; (ii) the MA holder for the first orphan medicinal product grants its consent; or (iii) if the MA holder of the orphan medicinal product is unable to supply sufficient quantities. MAs may also be granted for the same therapeutic indication in relation to products that are not similar. 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 and resulting regulatory exclusivity 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 and/or the Orphan Regulation, as applicable, to maintain a competitive position. If we do not obtain orphan drug designation and related regulatory exclusivity for our products that

do not have broad patent protection or if a competing product is determined to be "clinically superior" to any of our products that has secured orphan drug exclusivity, 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 regulatory 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 certain biologics and gene therapies, there may be some uncertainty regarding how similarity between product candidates designed to treat the same rare disease or condition may affect such product candidates' orphan drug regulatory exclusivities. For biologics and gene therapies, the FDA's determination of whether a drug is the same drug or a different drug will be based on the principal molecular structural features of the products. For gene therapy products, the FDA has stated in guidance that it generally intends to consider certain key features such as transgenes and vectors used in gene therapy products to be principal molecular structural features. The FDA has not yet proffered additional information on orphan drug sameness for gene therapy or similar products. 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, ROCTAVIAN and VIMIZIM products are regulated by the FDA as biologics under the Federal Food, Drug, and Cosmetic Act and the Public Health Service Act (the PHS Act). Biologics require the submission of a BLA and licensure 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 licensure of biological products that are demonstrated to be "biosimilar" to or "interchangeable" with an FDA-licensed biological product. A similar abridged MA process is available to biosimilar products in the EU. In particular, applicants for MAs of biosimilars are required to demonstrate through comprehensive comparability studies with the reference biological medicine that: a) their biological medicine is highly similar to the reference medicine, notwithstanding natural variability inherent to all biological medicines; and b) there are no clinically meaningful differences between the biosimilar and the reference medicine in terms of safety, quality and efficacy.

In the U.S., 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 in any given patient, and for a product that is administered more than once, that the risk of switching in terms of safety or diminished efficacy of alternating or 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 data exclusivity for reference products but such data exclusivity only blocks licensure of biosimilars relying on the product as a reference product; it will not prevent the licensure of the same product for the same or different indications that does not seek to rely on reference product data. In the EU, 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 biosimilar applications may be submitted and the reference product's data may be referenced but 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 as a result of Marketing Authorization Applications (MAAs) in the EU, as well as our product candidates that may be approved in the future, could be reference products for biosimilar marketing applications.

Changes in funding for the FDA, the EMA, other comparable regulatory authorities and other government agencies or government shutdowns could hinder the ability of such authorities and agencies to hire and retain key leadership and other personnel or otherwise prevent those authorities and 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 regulatory authorities and 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 or the EMA to timely review and approve INDs or MAAs for our product candidates may be hindered by a lack of resources and qualified personnel. In addition, funding of other regulatory authorities and 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 regulatory authorities and 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 authorities and 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.

Financial and Financing Risks

If we incur operating losses or are unable to sustain positive cash flows for a period longer than anticipated, we may be unable to continue our operations at planned levels and may 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 most years since our inception and there is no guarantee that we will achieve or maintain profitability in the future. 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 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, 2024, we had cash, cash equivalents and investments totaling $1.8 billion and debt obligations of $1.1 billion (undiscounted), which consisted of our 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes) and our 1.25% senior subordinated convertible notes due in 2027 (the 2027 Notes). The 2024 Notes matured on August 1, 2024 and substantially all holders of the 2024 Notes were repaid with cash, and the 2027 Notes, if not converted, will be required to be repaid in cash at maturity in May 2027. We will need cash not only to pay the ongoing interest due on the 2027 Notes during their term, but also to repay the principal amount of the 2027 Notes if not converted.

We may require additional financing to fund the repayment of the 2027 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;
- 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's 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 equity and/or equity-linked 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, 2024, we had $1.1 billion (undiscounted) principal amount of indebtedness, including $495.0 million (undiscounted) principal amount of indebtedness under the 2024 Notes and $600.0 million (undiscounted) principal amount of indebtedness under the 2027 Notes. The 2024 Notes matured on August 1, 2024 and substantially all holders of the 2024 Notes were repaid with cash. 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.

If we default under the 2027 Notes, the 2027 Notes could become immediately due and payable.

***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 2027 Notes, which, if not converted, will be required to be repaid in cash at maturity in May 2027. While we could seek to obtain additional third-party financing to pay for any amounts due in cash upon maturity of the 2027 Notes, we cannot be sure that such third-party financing will be available on commercially reasonable terms, if at all.

Manufacturing Risks

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

Prior to commercialization of our products, regulatory authorities must approve marketing applications that identify authorized manufacturing facilities operated by us or our contract manufacturers that are in compliance with cGMP requirements. In addition, our pharmaceutical manufacturing facilities are continuously subject to scheduled and unannounced regulatory inspections by the FDA, and other comparable EU and other national and international regulatory authorities, before and after product approval, to monitor and ensure compliance with cGMP and other regulations. Our manufacturing facilities in the U.S. are licensed for the manufacture of PALYNZIQ, ROCTAVIAN, ALDURAZYME, BRINEURA, NAGLAZYME, VIMIZIM, and VOXZOGO. Our manufacturing facility in Shanbally, Cork, Ireland is licensed for the manufacture of VIMIZIM and BRINEURA and packaging operations for VOXZOGO and PALYNZIQ. 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, national 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, national 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 product candidates to produce sufficient quantities at acceptable costs, we may be unable to support a clinical trial or be forced to terminate a program, or if we are unable to produce sufficient quantities of our products at acceptable costs, we may be unable to meet commercial demand, lose potential revenue, have reduced margins or be forced to terminate a program.**

Due to the complexity of manufacturing our product candidates and products, we may not be able to manufacture sufficient quantities. Our inability to produce enough of our product candidate at acceptable costs may result in the delay or termination of development programs. With respect to our commercial portfolio, we may not be able to manufacture our products successfully with a commercially viable process or at a scale large enough to support their respective commercial markets or at acceptable margins. For example, demand for VOXZOGO in certain markets outpaced our projections in recent quarters, and we have faced, and may in the future face, challenges meeting demand, requiring us to postpone planned entry into additional markets until VOXZOGO inventory levels increase or delay certain VOXZOGO development activities. As a result of such inventory constraints, we have lost, and may in the future lose, potential VOXZOGO revenues that may never be recouped and our VOXZOGO development program could be adversely impacted.

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.

Our gene therapy product and product candidates are based on relatively novel technology, which presents additional manufacturing risks in relation to our other, more traditional drug development programs. Gene therapy products are complex 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 gene therapy 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 gene therapy clinical studies in a timely manner, if at all, or commercializing our gene therapy products on a profitable basis, if at all.

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 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 currently rely on third parties for portions of the manufacture of each of our products. 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, supranational, national, 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, including inflation.

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.

If our Manufacturing, Marketing and Sales Agreement with Sanofi 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 Sanofi 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 Sanofi 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 Sanofi 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 Sanofi'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 Sanofi'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.

Risks Related to International Operations

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

A significant portion of the sales of our products are generated from countries other than the U.S., and we expect international markets will continue to be important for the sales of any products approved in the future. 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;
- geopolitical and economic instability, such as the instability caused by Russia's invasion of Ukraine;
- 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 non-U.S. 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. For example, Russia's invasion of Ukraine and the related impacts to Ukraine's infrastructure and healthcare system has significantly impacted our ability to provide our therapies to patients in Ukraine. Sanctions issued by the U.S. and other countries against Russia and Belarus in response to the attack on Ukraine and related counter-sanctions issued by Russia have made it very difficult for us to operate in Russia and may have a material adverse impact on our ability to sell our products and/or collect receivables from customers in Russia and Belarus.

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 revenues and profitability.

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 revenues in these countries.

We make a significant portion of our initial international sales of newly launched products through early access, special access or "named patient sales" programs in markets where we are not required to obtain regulatory approval before establishing these programs. For example, a significant portion of our international sales of VOXZOGO since the product's launch have been made through such programs. The specifics of the programs vary from country to country. Generally, special approval must be obtained to initiate such programs, and in some cases, special approval must be obtained for each patient. The approval normally

requires an application to national competent authorities in which the product is intended to be supplied or a lawsuit accompanied by evidence of medical need.

These programs are not well defined in some countries and are subject to changes in requirements, funding levels, unmet medical need and classification of the disease treated by our product. 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, requiring additional in-country testing 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 or official reimbursement 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, obtain and maintain a full product approval or official reimbursement, 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.

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 assets and liabilities, are exposed to changes in foreign exchange rates. As we operate in multiple foreign currencies, including the Euro, the Brazilian Real, the Russian Ruble, the Colombian Peso, the Argentine Peso and several other currencies, changes in those currencies relative to the U.S. Dollar (USD) will impact our revenues and expenses. If the USD 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 USD were to strengthen against another currency (as was the case for many currencies in 2022), 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 USD, changes in currency exchange rates between the USD 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.

U.S. export control and economic sanctions may adversely affect our business, financial condition and operating results. Moreover, compliance with such regulatory requirements may increase our costs and negatively impact our ability to sell our products and collect cash from customers.

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 hinder our 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. For example, sanctions issued by the U.S. and other jurisdictions against Russia and Belarus in response to the invasion of Ukraine have made it very difficult for us to operate in Russia and may have a material adverse impact on our ability to sell our products and/or collect receivables from customers in Russia and Belarus. Moreover, if we fail to comply with these laws and regulations, we could be subject to substantial civil or criminal penalties, including the possible loss of export or import privileges and fines.

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, the general license will be renewed in the future or we will remain in compliance with the general license. 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.

Moreover, U.S. export control and economic sanctions may make operating in certain countries more difficult and expensive. For example, we may be unable to find distributors or financial institutions willing to facilitate the sale of our products and collection of cash from such sales in a cost-effective manner, if at all.

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 U.S. 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 non-U.S. government 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 non-U.S. government 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 independent 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.

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.

Intellectual Property Risks

***If we are unable to protect our intellectual property, we may not be able to compete effectively or preserve our market shares.**

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) has 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.
- 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.

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.

In the EU, materials we submit to the EMA in connection with our clinical trials that were traditionally regarded as confidential, proprietary information, such as study protocols, information regarding manufacturing methods and controls, and intermediate data analyses, are now subject to public disclosure. Moreover, clinical trial data submitted to the EMA in our MAAs are also available to the public. We are only permitted to redact from public disclosures commercially confidential information, a standard which is construed narrowly and subject to the interpretation and final decision of the EU regulatory authorities. EU regulations have resulted and will continue to result in the EMA’s public disclosure of certain of our proprietary information related to recently completed and future clinical trials and MAA submissions. The move toward public disclosure of such development information 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, increasing scrutiny of our product candidates and products, and enabling competitors to use our clinical trial information and data to gain approvals for their own products.

Competitors or other third parties may interfere with our patent process in a variety of ways. Competitors or other third parties 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 or other third parties may also claim that we are infringing on their patents and therefore we cannot practice our technology. Competitors or other third parties 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, any such party 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. Moreover, follow-on manufacturers, including generic and biosimilar manufacturers, may use litigation and regulatory means to obtain approval for generic, biosimilar, or other follow-on versions of our products notwithstanding our filed patents or patent applications.

If we are unable to protect our intellectual property, third parties could develop competing products, which could adversely affect our revenues 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 and other third parties continually seek intellectual property protection for their technology. Several of our products, such as ROCTAVIAN, and development programs, 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 other third parties or that we will not infringe intellectual property rights of competitors or other third parties 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 their 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 others 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. For example, under the Bayh-Dole Act which only applies to patents for inventions generated from federally funded research, the U.S. Department of Commerce may allow the government to use "march-in" rights for prescription drug patents as a means to control prices.

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.

Risks Related to Ownership of Our Securities

***Our stock price has been and may in the future be volatile, and an investment in our stock could suffer a decline in value.**

Our stock price has been and may in the future be volatile. Our valuation and stock price may 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 has fluctuated, and in the future could fluctuate, due to factors including:

- product sales and profitability of our products;
- manufacturing, supply or distribution of our product candidates and 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 described above in this Risk Factors section 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 periodic orders for our products by governments in certain countries;

- changes in company assessments or financial estimates by securities analysts;
- certain actions by activist investors that may be threatened or commenced against us;
- acquisitions of products, businesses, or other assets;
- industry, financial analyst, or investor reaction to our public announcements; and
- sales of our shares of stock by us, our significant stockholders, or members of our management or Board of Directors.

Furthermore, the stock markets have recently experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many companies. In some cases, these fluctuations have been unrelated or disproportionate to the operating performance of those companies. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. For example, in September 2020, after a substantial drop in our stock price that followed an announcement providing a regulatory update regarding ROCTAVIAN, we and certain of our officers were sued in a putative class action lawsuit alleging violations of the federal securities laws for allegedly making materially false or misleading statements. In addition, in October 2021, after a drop in our stock price that followed an announcement providing a regulatory update regarding BMN 307, we and certain of our current and former officers were sued in a putative class action lawsuit alleging violations of the federal securities laws for allegedly making materially false or misleading statements. We may be the target of additional litigation of this type in the future as well. Securities litigation against us could result in substantial costs and divert our management's time and attention from other business concerns, which could harm our business.

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 2027 Notes will dilute the ownership interest of existing stockholders, including holders who had previously converted their 2027 Notes, or may otherwise depress the price of our common stock.**

The conversion of some or all of the 2027 Notes will dilute the ownership interests of existing stockholders. 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 2027 Notes may encourage short selling by market participants because the conversion of the 2027 Notes could be used to satisfy short positions, or anticipated conversion of the 2027 Notes into shares of our common stock could depress the price of our common stock.

***The fundamental change repurchase feature of the 2027 Notes may delay or prevent an otherwise beneficial attempt to take us over.**

The terms of the 2027 Notes require us to offer to repurchase the 2027 Notes in the event of a fundamental change (as defined in the indenture governing the 2027 Notes). A takeover of us would trigger options by the respective holders of the 2027 Notes to require us to repurchase such 2027 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 2027 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.

Our amended and restated bylaws designate the Court of Chancery of the State of Delaware and the federal district courts of the U.S. as the exclusive forums 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 the following types of actions or proceedings under Delaware statutory or common law:

- any derivative claim or cause of action brought on our behalf;
- any claim or cause of action for breach of a fiduciary duty owed by any current or former director, officer or other employee of BioMarin to us or our stockholders;
- any claim or cause of action against us or any of our current or former 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; any claim or cause of action seeking to interpret, apply, enforce or determine the validity of our restated certificate of incorporation or our amended and restated bylaws;
- any claim or cause of action as to which the General Corporation Law of the State of Delaware confers jurisdiction to the Court of Chancery of the State of Delaware; and
- any claim or cause of action against us or any of our current or former directors, officers or other employees that is governed by the internal affairs doctrine.

This exclusive-forum provision would not apply to suits brought to enforce a duty or liability created by the Securities Act of 1933, as amended, the Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction. In addition, our amended and restated bylaws provide that the federal district courts of the U.S. of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act.

While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated bylaws. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions 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 either of our exclusive forum provisions to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business. Our amended and restated bylaws further provide 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 provisions.

General Risk Factors

We depend upon our key personnel and our ability to attract and retain qualified 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 a significant portion of our workforce or any member of our senior management or the inability to hire or retain qualified personnel could adversely affect our ability to execute our business plan and harm our operating results.

Because of the specialized 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. In November 2023, we announced the retirement of Jean-Jacques Bienaimé, our then-current President and Chief Executive Officer, and the appointment of Alexander Hardy as President and Chief Executive Officer, each effective December 1, 2023. If Mr. Hardy's succession as President and Chief Executive Officer is not managed successfully, including his ability to lead a team that can effectively implement our strategic plans, it could disrupt our business and affect our financial condition and operating results. Additionally, in March 2024, we announced that Cristin Hubbard will succeed Jeffrey Ajer, our Executive Vice President and Chief Commercial Officer, effective May 20, 2024. The recent changes in our management team could cause retention and morale concerns among current employees, as well as operational risks.

The competition for qualified personnel in the pharmaceutical field is intense, and there is a limited pool of qualified potential employees to recruit. Recently, like many other employers in the U.S., we experienced increased employee turnover. Due

to the intense competition for talent, 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. Additionally, we cannot be sure that the compensation costs of doing so will not adversely affect our operating results, and we may not be able to hire and train employees quickly enough to meet our needs. If we fail to retain employees and effectively manage our hiring needs, our efficiency, ability to meet forecasts, employee morale, productivity, and the success of our strategic plans could suffer, which may have an adverse effect on our business, financial condition, and operating results.

***Our success depends on our ability to manage our growth.**

VOXZOGO addresses larger patient populations than most of our other products, and product candidates that we are currently developing or may license or acquire in the future may be intended for similarly larger patient populations than we have historically targeted. In order to continue development of such product candidates and marketing of products with larger markets, we will need to continue expanding 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. For example, strong demand for VOXZOGO in certain markets outpaced our projections in recent quarters, and we faced, and may in the future face, challenges meeting our estimates of VOXZOGO demand. Our staff, financial resources, systems, procedures or controls may be inadequate to support our operations and may increase our exposure to regulatory, competitive, and corruption risks and our management may be unable to manage successfully current or future market opportunities or our relationships with customers and other third parties.

In addition, there is no guarantee that our research and development prioritization activities, including the acceleration or discontinuation of certain programs, will generate their expected benefits and the costs associated with such activities may be greater than anticipated. Such activities may also adversely affect our internal programs and initiatives as well as our ability to recruit and retain skilled and motivated personnel. If we are unable to realize the expected benefits of such activities, then our operating results and financial condition may be materially and adversely affected.

New tax laws or regulations that are enacted or existing tax laws and regulations that are interpreted, modified or applied adversely to us or our customers may have a material adverse effect on our business and financial condition.

New tax laws or regulations could be enacted at any time, and existing tax laws or regulations could be interpreted, modified or applied in a manner that is adverse to us or our customers, which could adversely affect our business and financial condition. For example, the Tax Cuts and Jobs Act, the Coronavirus Aid, Relief, and Economic Security Act and the Inflation Reduction Act enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to such legislation may affect us, and certain aspects of such legislation could be repealed or modified in future legislation. Among other changes, the Tax Cuts and Jobs Act amended the Code to require that, for tax years beginning after December 31, 2021, certain research and experimental expenditures be capitalized and amortized over five years if incurred in the United States or fifteen years if incurred in foreign jurisdictions. Although the U.S. Congress has considered legislation that would defer, modify, or repeal the capitalization and amortization requirement, there is no assurance that such changes will be made. If the requirement is not deferred, repealed or otherwise modified, it may increase our cash tax. In addition, it is uncertain if and to what extent various states will conform to federal tax laws. Any future tax legislation could increase our U.S. tax expense and could have a material adverse impact on our business and financial condition.

Moreover, changes in the tax laws of jurisdictions in which we conduct business could arise, including as a result of the base erosion and profit shifting (BEPS) project that is being led by the Organization for Economic Co-operation and Development (OECD), and other initiatives led by the OECD or the EC. For example, the OECD, which represents a coalition of member countries including the U.S. and other countries in which we have operations, is working on proposals, commonly referred to as “BEPS 2.0”, which, if and to the extent implemented, would make important changes to the international tax system. These proposals are based on two “pillars”, Pillar One focuses on the allocation of taxing rights in respect of certain profits of multinational enterprises with annual global revenue above 20 billion euros and profitability above 10% to the jurisdictions within which they carry on business (based on the thresholds, we currently expect to be outside the scope of the Pillar One proposals, but could fall within their scope in the future) and Pillar Two imposes a minimum effective tax rate of 15% on certain multinational enterprises that have consolidated revenues of at least 750 million euros in at least two out of the last four years (based on the thresholds, we currently expect that we are likely to fall within the scope of the Pillar Two proposals). A number of countries in which we conduct business have enacted with effect from January 1, 2024, or are in the process of enacting, core elements of Pillar Two rules. The OECD has issued administrative guidance providing transition and safe harbor rules around the implementation of Pillar Two. We are monitoring developments and evaluating the impacts these new rules will have on our tax rate, including eligibility to qualify for these safe harbor rules. It is not uncommon for taxing authorities in different countries to have conflicting views, for instance, with respect to, among other things, the manner in which the arm’s length standard is applied for transfer pricing purposes, or with respect to the valuation of intellectual property. If tax authorities successfully challenge our transfer prices as not reflecting arm’s length transactions, they could require us to adjust our transfer prices and thereby reallocate our income to reflect these revised transfer prices, resulting in a higher tax liability. In addition, if a country from which income is reallocated does not agree with the reallocation, both that country and the other country to which the income was allocated could tax the same income, potentially resulting in double taxation. If tax authorities were to allocate income to a higher tax jurisdiction, subject our income to double

taxation or assess interest and penalties, it would increase our consolidated tax liability, which could adversely affect our business, financial condition, results of operations and cash flows.

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, reputation, 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. As previously disclosed, we received a subpoena from the U.S. Department of Justice requesting that we produce certain documents regarding our sponsored testing programs relating to VIMIZIM and NAGLAZYME. We have produced documents in response to the subpoena and are cooperating fully, but there is no assurance that such sponsored testing programs, or our other operations or programs, will not be found to violate such laws.

Federal and state false claims laws, including the civil False Claims Act and the Civil Monetary Penalties Law, 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, among other things, 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, federal and state healthcare legislation have 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 non-U.S. 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 U.S., state privacy laws and regulations impose restrictive requirements regulating the use and disclosure of health information and other sensitive personal information that is not subject to HIPAA. For example, California enacted the California Consumer Privacy Act (CCPA), which took effect on January 1, 2020. The CCPA gives California consumers expanded rights to access and delete their personal information, opt out of certain personal information sales, 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 was expanded substantially on January 1, 2023 when the California Privacy Rights Act of 2020 (CPRA) took effect and amended the CCPA. Following the CPRA amendments, the CCPA, among other things, gives consumers the ability to limit use of information deemed to be sensitive, increase the maximum penalties for violations concerning consumers under age 16, expands an individual's private right of action and establishes the California Privacy Protection Agency to implement and enforce the new law and impose administrative fines.

Other U.S. states have recently adopted consumer data protection and privacy laws, and more U.S. states may do so in the future. This creates the potential for a patchwork of overlapping but different state laws and 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,

financial condition, and results of operations. Many other states are considering proposed comprehensive data privacy legislation and all 50 states have passed some form of legislation relating to privacy or cybersecurity.

Aspects of the CCPA, CPRA and similar laws in other states and their interpretation and enforcement remain uncertain. The potential effects of these laws are far-reaching and may require us to modify our data processing practices and policies and to incur substantial costs and expenses in an effort to comply. Complying with these or other similar laws, regulations, amendments to or re-interpretations of existing laws and regulations, and contractual or other obligations relating to privacy, data protection, data transfers, data localization, or information security may require us to make changes to our services to enable us or our customers to meet new legal requirements, incur substantial operational costs, modify our data practices and policies, and restrict our business operations. Any actual or perceived failure by us to comply with these laws, regulations, or other obligations may lead to significant fines, penalties, regulatory investigations, lawsuits, significant costs for remediation, damage to our reputation, or other liabilities.

The European Regulation 2016/679, known as the General Data Protection Regulation (GDPR), as well as EEA Member State legislations supplementing such regulation, apply to the processing of personal data of individuals located in the EEA, including health-related information, by companies located in the EEA, or in certain circumstances, by companies located outside of the EEA. These laws impose strict obligations on the ability to collect, record, store, disclose, use and transmit personal data, including health-related information. These include several requirements relating to (i) obtaining, in some situations, the informed 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). Switzerland has adopted similar restrictions.

The GDPR and other European data protection laws generally restrict the transfer of personal information from Europe, including the EEA and Switzerland, to the U.S. and most other countries unless the U.S. companies participate in the EU-U.S. Data Privacy Framework in accordance with the EC's adequacy decision adopted on July 10, 2023, or have implemented specific safeguards to protect the transferred personal information. U.S. companies can join the EU-U.S. Data Privacy Framework by committing to comply with a detailed set of privacy obligations and U.S. companies that do not fall under the EU-U.S. Data Privacy Framework must implement certain specific safeguards. One of the primary safeguards allowing U.S. companies to import personal information from the EEA has been the EC’s Standard Contractual Clauses (SCCs). However, the Court of Justice of the EU (CJEU) issued a decision that called into question whether the SCCs can lawfully be used for transfers of personal information from Europe to the United States or most other countries. After the mentioned CJEU judgment, new sets of SCCs were published on June 4, 2021. Most importantly, the use of SCCs does not any longer automatically ensure compliance with the GDPR. Instead, companies remain required to conduct a data transfer impact assessment for each transfer, which adds a compliance burden.

Potential pecuniary fines for noncompliance with the GDPR 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 has increased our compliance costs. The EU regulations that make certain materials we submit to the EMA in connection with our clinical trials subject to public disclosure have increased the risk that we may unintentionally disclose personal information protected under the GDPR and thereby incur associated penalties and suffer reputational damage.

In addition to the U.S. and European countries, other countries in which we operate have also enacted data privacy laws or may do so in the future. For example, Brazil’s General Data Protection Law (LGPD), which is modeled on the GDPR, took effect in 2020.

Substantial new laws and regulations affecting compliance have also been adopted in the U.S. and certain non-U.S. 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 (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other health care professionals (such as physicians assistants and nurse practitioners), and teaching hospitals, as well as investment and ownership interests held by such physicians and their immediate family members during the preceding calendar year. In addition, there has been a recent trend of increased state regulation of payments made to physicians. Certain states and/or local jurisdictions mandate implementation of compliance programs, compliance with the Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the Pharmaceutical Research and Manufacturers of America (PhRMA) Code on Interactions with Healthcare Professionals, the registration of pharmaceutical sales representatives and/or the tracking and reporting of gifts, compensation and other remuneration to physicians, marketing expenditures, and drug pricing. Likewise, in many non-U.S. countries there is an increasing focus on the relationship between drug companies and healthcare practitioners. Recently enacted non-U.S. legislation creates reporting obligations on payments, gifts and benefits made to these professionals. Outside the U.S., interactions between pharmaceutical companies and health care professionals are also governed by strict laws, such as national anti-bribery laws of European countries, national sunshine rules, regulations, industry self-regulation codes of conduct and physicians’ codes of professional conduct. 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 authorities 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 significant criminal, civil or administrative sanctions, including damages, fines, disgorgement, imprisonment, contractual damages, reputational harm, public reprimands, 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.

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.

In the EU, new rules on liability of defective products were proposed on September 28, 2022. If adopted, these rules will make it easier for patients to claim damages for defective products, for example by alleviating their burden of proof.

***We rely significantly on information technology systems and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively and have a material adverse effect on our business, reputation, financial condition, and results of operations.**

We rely significantly on our information technology systems, including enterprise resource planning (ERP), production management, and other information systems, 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 (whether intentional or inadvertent) of that technology, including cybersecurity incidents or attacks, could harm our ability to operate our business effectively.

We are currently implementing a new global ERP system, which will replace existing operating and financial systems. The preparation and implementation of a new ERP system has required, and will continue to require, significant investment of capital and human resources. Our results of operations could be adversely affected if we continue to experience delays or cost overruns during the implementation process, or if the ERP system or associated process changes do not give rise to the benefits that we expect. Potential failure or flaws in the new ERP system may pose risks to our ability to operate successfully and efficiently and failure to implement the appropriate internal controls with respect to the new ERP system may result in the system producing inaccurate or unreliable information. Any disruptions, delays or deficiencies in the design or implementation of the new ERP system or related internal controls, or in the performance of legacy information technology systems, could result in potentially much higher costs than we had incurred and adversely affect our ability to effectively fulfill contractual obligations, file related government reports in a timely manner, operate and manage our business or otherwise affect our controls environment. Any of these consequences could have an adverse effect on our results of operations and financial condition.

In addition, our technology systems, including our cloud technologies, continue to increase in multitude and complexity, making them potentially vulnerable to breakdown, cyberattack and other disruptions. Potential problems and interruptions associated with the implementation of new or upgraded technology systems or with maintenance or adequate support of existing systems could disrupt or reduce the efficiency of our operations and expose us to greater risk of security breaches. 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 or unavailability of these systems or those of any third parties in our supply chain or on whom we otherwise depend, have occurred in the past and may affect our ability in the future to manage and maintain our operations, inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations.

As part of our business, we collect, store, and transmit large amounts of confidential information, proprietary data, intellectual property, and personal data. The information and data processed and stored in our technology systems, and those of our research collaborators, CROs, contract manufacturers, suppliers, distributors, or other third parties on whom we depend on to operate our business, may be vulnerable to loss, damage, denial-of-service, unauthorized access or misappropriation. Data security incidents may be the result of unauthorized or unintended activity (or lack of activity) by our employees, contractors, or others with authorized access to our network or malware, hacking, business email compromise, phishing, ransomware or other cyberattacks directed by third parties. While we have implemented measures to protect our information and data stored in our technology systems and those of the third parties with whom we work, our efforts may not be successful.

We have experienced and may continue to experience cybersecurity incidents, although to our knowledge we have not experienced any material incident or interruption to date. If such a significant event were to occur, it could result in a material disruption of our development programs and commercial operations, including due to a loss, corruption or unauthorized disclosure of our trade secrets, personal data or other proprietary or sensitive information. Further, these cybersecurity incidents can lead to the public disclosure of personal information (including sensitive personal information) of our employees, clinical trial patients and others and result in demands for ransom or other forms of blackmail. Such attacks, including phishing attacks and attempts to misappropriate or compromise confidential or proprietary information or sabotage enterprise IT systems, are of ever-increasing levels of sophistication and are made by groups and individuals with a wide range of motives (including industrial espionage) and expertise, including by organized criminal groups, “hacktivists”, nation states and others. Moreover, the costs to us to investigate and mitigate cybersecurity incidents could be significant. For example, the loss of clinical trial data could result in delays in our product development or regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Any security breach that results in the unauthorized access, use or disclosure of personal data may require us to notify individuals, governmental authorities, credit reporting agencies, or other parties pursuant to privacy and security laws and regulations or other obligations. Such a security compromise could harm our reputation, erode confidence in our information security measures, and lead to regulatory scrutiny. To the extent that any disruption or security breach resulted in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential, proprietary or personal information, we could be exposed to a risk of loss, enforcement measures, penalties, fines, indemnification claims, litigation and potential civil or criminal liability, which could materially adversely affect our business, financial condition and results of operations.

Not all our contracts contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

Further, the SEC has adopted new rules that require us to provide greater disclosures around proactive security protections that we employ and reactive issues (e.g., security incidents). Any such disclosures, including those under state data breach notification laws, can be costly, and the disclosures we make to comply with, or the failure to comply with, such requirements could lead to adverse consequences.

If a natural disaster, terrorist or criminal activity or other unforeseen event caused significant damage to our facilities or those of our third-party manufacturers and suppliers or significantly disrupted our operations or those 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.

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 the ability for us or our third-party manufacturers to manufacture our products and product candidates. Our Galli Drive facility, located in Novato, California, is currently our only manufacturing facility for ALDURAZYME, NAGLAZYME, VOXZOGO 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 ongoing ROCTAVIAN clinical development activities and commercial demand for ROCTAVIAN. 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, 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 revenues could be seriously impaired.

Moreover, other unforeseen events, such as power outages, could significantly disrupt our operations or those of our third-party manufacturers and suppliers, which could result in damage to our facilities and significant delays in the manufacture of our products and adversely impact our commercial operations and revenues. 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.

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, or foreign currency exchange rates, natural disasters, geopolitical instability resulting from war, terrorism and other violence, such as the instability caused by Russia's invasion of Ukraine, effects of potential global public health threats and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets and volatility and disruptions in the equity and debt markets. For instance, COVID-19 previously adversely affected our ability to source materials and supplies. Inflation (such as that recently observed in the U.S. and elsewhere) has increased our business costs and could become more significant in the future, and 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 counterparties 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 revenues 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, which could limit our ability to obtain sufficient materials and supplies necessary for production of our therapies. 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.

Additionally, effects of any pandemic or other global public health threat on all aspects of our business and operations and the duration of such effects are highly uncertain and difficult to predict. For instance, a global pandemic could result in significant disruption of global financial markets, which could reduce our ability to access capital and could negatively affect our liquidity and the liquidity and stability of markets for our common stock and Notes. In addition, a recession, further market correction or depression resulting from a future global public health threat could materially adversely affect our business and the value of our common stock and Notes.

To the extent macroeconomic conditions continue to adversely affect our business and financial results, they may also have the effect of heightening many of the other risks described in this Risk Factors section, such as those relating to our conducting a significant amount of our sales and operations outside of the U.S., exposure to changes in foreign exchange rates, our need to generate sufficient cash flows to service our indebtedness and finance our operations and the volatility of our stock price.

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

Rule 10b5-1 Trading Plans

During the three months ended June 30, 2024, none of our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated any contracts, instructions or written plans for the purchase or sale of BioMarin securities. In addition, in Item 5 of Part II of our Quarterly Report on Form 10-Q for the quarter ended March 31, 2024, we inadvertently omitted the disclosure regarding the termination of the Rule 10b5-1 trading arrangement (as defined in Item 408(a) of Regulation S-K under the Exchange Act) by Henry J. Fuchs, M.D., President of Worldwide Research & Development, on March 5, 2024. Such Rule 10b5-1 trading arrangement, which details were previously disclosed in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, was adopted on August 17, 2023 with an expiration date of November 15, 2024, and provided for the sale of up to 64,659 shares of our common stock.

Litigation

On April 4, 2024, Plaintiff Aaron Jones ("Plaintiff"), a purported stockholder of ours, filed a putative class action complaint ("Complaint") in the Court of Chancery of the State of Delaware ("Court") against the Company and the members of our Board of Directors, as well as Elliott Investment Management L.P., Elliott Associates, L.P., and Elliott International, L.P. (together, the "Elliott Parties" and collectively, the "Defendants") under the caption Jones v. BioMarin Pharmaceutical, Inc., et al., C.A. No. 2024-0360-JTL (the "Action"). Plaintiff alleged that certain provisions in a Cooperation Agreement dated December 20, 2023 between the Company and the Elliott Parties, as defined and described in the Company's prior Form 8-K, filed with the SEC on December 20, 2023, were invalid under Section 141(a) of the Delaware General Corporation Law insofar as they allegedly required the Company's Board to (i) cap the size of the Board of Directors for a certain period of time (the "Board Size Provision") and (ii) "recommend that the stockholders of the Company vote to elect" certain directors in connection with the Company's 2024 annual meeting (the "Recommendation Provision"). The Defendants believe that the allegations of the Complaint were meritless, deny those allegations, and deny that any violation of applicable law has occurred. However, solely to minimize expenses and distraction and to avoid the uncertainty of any litigation, on April 11, 2024, the Elliott Parties executed a Waiver Letter, pursuant to which, among other things, the Elliott Parties irrevocably waived (i) any and all obligations of the Company pursuant to the Board Size Requirement and (ii) any and all obligations of the Company pursuant to the Recommendation Provision to the extent it is superseded by the Board's fiduciary obligations under Delaware law.

On April 16, 2024, the parties entered into a proposed Stipulation and Order Dismissing the Action as Moot and Retaining Jurisdiction to Determine Plaintiff's Counsel's Application for an Award of Attorneys' Fees and Expenses (the "Stipulation and Proposed Order"), pursuant to which the Court would retain jurisdiction regarding any application Plaintiff may make for an award of attorneys' fees. The Court entered the Stipulation and Proposed Order the same day, and retained jurisdiction to approve a form of notice concerning attorneys' fees payable to Plaintiff in connection with the Waiver Letter. The Company subsequently agreed to pay $600,000 in attorneys' fees and expenses in full satisfaction of any and all claims by Plaintiff and all of his counsel for fees and expenses in the Action.

On August 1, 2024, the Court entered an order closing the Action, subject to the Company filing an affidavit with the Court confirming that this notice has been issued.

In entering the order, the Court was not asked to review, and did not pass judgment on, the payment of the attorneys' fees and expenses or their reasonableness. Plaintiff's counsel are Kurt M. Heyman and Aaron M. Nelson of Heyman Enerio Gattuso & Hirzel LLP, (302) 472-7300. Counsel to the Company and the Board of Directors is Blake Rohrbacher of Richards, Layton & Finger, P.A., (302) 651-7700. Counsel to the Elliott Parties is Michael A. Barlow of Quinn Emanuel Urquhart & Sullivan, LLP, (302) 302-4000.

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 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.
2.3	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 December 21, 2022 as Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.1*	Amendment, dated as of June 8, 2024, to the Consulting Agreement by and between BioMarin Pharmaceutical Inc. and Jean-Jacques Bienaimé, dated October 30, 2023.
10.2*	BioMarin Pharmaceutical Inc. Summary of Independent Director Compensation.
31.1*	Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
32.1*+	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.
101.INS	XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase
101.LAB*	Inline XBRL Taxonomy Extension Labels Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Link Document
104	XBRL tags for the cover page from the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2024, are embedded within the Inline XBRL document.

* Filed herewith

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

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language):

(i) Condensed Consolidated Balance Sheets as of June 30, 2024 and December 31, 2023, (ii) Condensed Consolidated Statements of Comprehensive Income for the three and six months ended June 30, 2024 and 2023, (iii) Condensed Consolidated Statement of Stockholders’ Equity for the three and six months ended June 30, 2024 and 2023, (iv) Condensed Consolidated Statements of Cash Flows for the six months ended June 30, 2024 and 2023, 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 5, 2024

By /S/ BRIAN R. MUELLER

Brian R. Mueller
Executive Vice President, Finance & Chief Financial Officer
(Principal Financial Officer)

AMENDMENT TO CONSULTING AGREEMENT

This Amendment (this "**Amendment**"), entered into and effective as of June 8, 2024 (the "**Effective Date**"), is made by and between BioMarin Pharmaceutical Inc. ("**BioMarin**"), a Delaware corporation, located at 105 Digital Drive, Novato, CA 94949 and Jean-Jacques Bienaime ("**Provider**").

WHEREAS, BioMarin and Provider entered into a Consulting Agreement on October 30, 2023, effective as of December 1, 2023 (the "**Consulting Agreement**")

WHEREAS, BioMarin and Provider desire to amend the Consulting Agreement pursuant to this Amendment;

NOW, THEREFORE, in consideration of the above recitals and mutual covenants contained herein, BioMarin and Provider agree as follows:

1. The final sentence of Section 2.3.1 of the Consulting Agreement is hereby amended and restated to read as follows:

> "**Change in Control**" means any one or more of the following events: (i) a merger, consolidation, share exchange, business combination, issuance of securities, direct or indirect acquisition of securities, tender offer, exchange offer or other similar transaction as a result of which any one person, or more than one person acting as a group, acquires ownership of shares of BioMarin's voting stock that, together with other such stock held by such person or group, constitutes more than fifty percent (50%) of the total voting power of all outstanding classes of voting stock of BioMarin or the continuing or surviving corporation if BioMarin is not the continuing or surviving corporation in such transaction, or (ii) a sale of all or substantially all of the assets of BioMarin; provided, however, that an event will constitute a Change in Control only if it is also a change in ownership or effective control of the corporation or a change in the ownership of a substantial portion of the assets of the corporation, as defined in Treas. Reg. § 1.409A-3(i)(5).

2. Counterparts. This Amendment may be signed in counterparts, each of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument.

3. Ratification. All other provisions of the Consulting Agreement remain unchanged and are hereby ratified by BioMarin and Participant.

[Remainder of the page intentionally left blank]

IN WITNESS WHEREOF, the parties hereto, intending to be legally bound hereby, have caused this Amendment to be executed and delivered by their proper and duly authorized officers effective as of the Effective Date.

ACCEPTED AND AGREED TO:

Jean-Jacques Bienaime		**BioMarin Pharmaceutical Inc.**	
By:	/s/ Jean-Jacques Bienaime	By:	/s/ G. Eric Davis
Date:	June 7, 2024	Name:	G. Eric Davis
		Its:	Chief Legal Officer
		Date:	June 8, 2024

BioMarin Pharmaceutical Inc.

Summary of Independent Director Compensation

The summary below sets forth the compensation received by independent directors serving on the Board of Directors (the "Board") of BioMarin Pharmaceutical Inc. (the "Company"). This summary is consistent with what was reported in the Company's proxy statement previously filed with the Securities and Exchange Commission on April 9, 2024. All independent directors are entitled to receive a combination of annual cash retainers and restricted stock unit ("RSU") grants as summarized below as compensation for their service on the Company's Board and Board committees.

Cash Compensation

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

Director Position	Annual Cash Compensation
Compensation to All Independent Directors	
All Independent Directors	$ 65,000
Elements of Compensation in Addition to Director Membership Retainer	
Independent Chair of the Board (if applicable)	$ 80,000
Lead Independent Director (if applicable)	$ 65,000
Audit Committee Member	$ 13,500
Audit Committee Chair (premium in addition to committee membership retainer)	$ 13,000
Compensation Committee Member	$ 10,000
Compensation Committee Chair (premium in addition to committee membership retainer)	$ 10,000
Corporate Governance and Nominating Committee Member	$ 10,000
Corporate Governance and Nominating Committee Chair (premium in addition to committee membership retainer)	$ 10,000
Science and Technology Committee Member	$ 10,000
Science and Technology Committee Chair (premium in addition to committee membership retainer)	$ 10,000
Strategic and Operating Review Committee Member	$ 13,500
Strategic and Operating Review Committee Chair (premium in addition to committee membership retainer)	$ 13,500

Equity Compensation

Annual Award

On the date of our annual meeting of stockholders for a given year, each re-elected independent director receives a RSU grant valued at $400,000, based on the Black-Scholes model valuation using a 30-day trailing average closing price of the Company's common stock. The shares of common stock subject to the RSUs vest in full on the date immediately prior to the date of the Company's next regular annual meeting of stockholders (approximately on the one-year anniversary of the grant date), subject to each respective director providing services to the Company through the vesting date.

New Independent Directors

Upon election or appointment, a new independent director will receive an RSU grant on the same terms as the annual award, pro-rated for amount and vesting to the nearest quarter for the time such new director will serve prior to the Company's next annual meeting of stockholders.

Exhibit 31.1

CERTIFICATION

I, Alexander Hardy, 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.

Date: August 5, 2024

/S/ ALEXANDER HARDY

Alexander Hardy
President & Chief Executive Officer

Exhibit 31.2

CERTIFICATION

I, Brian R. Mueller 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.

Date: August 5, 2024

/S/ BRIAN R. MUELLER

Brian R. Mueller
Executive Vice President, Finance &
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, Alexander Hardy and Brian R. Mueller, 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, 2024, 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 Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of BioMarin Pharmaceutical Inc.

/s/ ALEXANDER HARDY

Alexander Hardy
President & Chief Executive Officer

Date: August 5, 2024

/s/ BRIAN R. MUELLER

Brian R. Mueller
Executive Vice President, Finance &
Chief Financial Officer

Date: August 5, 2024

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.