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

Form 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2025

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: 192,114,344 shares of common stock, par value $0.001, outstanding as of October 23, 2025.

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, 2024, which was filed with the Securities and Exchange Commission (the SEC) on February 24, 2025. 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

- Our success depends on our ability to manage our growth and execute our corporate strategy.
- 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.
- We have in the past and may in the future pursue acquisitions of other companies or businesses, which could divert our management's attention, fail to achieve the anticipated benefits and/or expose us to other risks or difficulties.
- 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.
- 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.
- 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.
- 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.
- Changes in methods of treatment of disease or failure of our products to gain acceptance by patients or the medical community could negatively impact demand for our products and adversely affect revenues.
- 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.

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 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.

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.
- Global trade issues and changes in and uncertainties with respect to trade policies and export regulations, including import and export license requirements, trade sanctions, tariffs and international trade disputes, could adversely impact our business and operations, and reduce the competitiveness of our products and services relative to local and global competitors.

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 (LOSS)

Three and Nine Months Ended September 30, 2025 and 2024

(In thousands, except per share amounts)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
REVENUES:				
Net product revenues	$ 760,812	$ 733,867	$ 2,308,438	$ 2,073,811
Royalty and other revenues	15,321	11,873	38,250	32,791
Total revenues	776,133	745,740	2,346,688	2,106,602
OPERATING EXPENSES:				
Cost of sales	140,085	188,457	441,733	444,096
Research and development	409,478	184,901	729,517	573,675
Selling, general and administrative	268,415	253,480	706,810	742,418
Intangible asset amortization	4,847	5,009	14,540	33,606
Gain on sale of nonfinancial assets	—	—	—	(10,000)
Total operating expenses	822,825	631,847	1,892,600	1,783,795
INCOME (LOSS) FROM OPERATIONS	(46,692)	113,893	454,088	322,807
Interest income	17,854	18,053	55,694	57,203
Interest expense	(2,579)	(2,968)	(8,121)	(10,089)
Other income, net	5,093	5,463	7,972	2,203
INCOME (LOSS) BEFORE INCOME TAXES	(26,324)	134,441	509,633	372,124
Provision for income taxes	4,420	28,361	114,159	70,208
NET INCOME (LOSS)	$ (30,744)	$ 106,080	$ 395,474	$ 301,916
EARNINGS (LOSS) PER SHARE, BASIC	$ (0.16)	$ 0.56	$ 2.06	$ 1.59
EARNINGS (LOSS) PER SHARE, DILUTED	$ (0.16)	$ 0.55	$ 2.04	$ 1.56
Weighted average common shares outstanding, basic	192,032	190,429	191,639	189,806
Weighted average common shares outstanding, diluted	192,032	197,147	196,893	196,683
COMPREHENSIVE INCOME (LOSS)	$ (20,116)	$ 83,931	$ 299,884	$ 335,354

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

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

September 30, 2025 and December 31, 2024

(In thousands, except share amounts)

	September 30, 2025	December 31, 2024 [1]
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 1,250,108	$ 942,842
Short-term investments	227,731	194,864
Accounts receivable, net	790,266	660,535
Inventory	1,382,173	1,232,653
Other current assets	204,265	201,533
Total current assets	3,854,543	3,232,427
Noncurrent assets:		
Long-term investments	512,937	521,238
Property, plant and equipment, net	1,038,187	1,043,041
Intangible assets, net	233,112	255,278
Goodwill	196,199	196,199
Deferred tax assets	1,509,109	1,489,366
Other assets	270,781	251,391
Total assets	$ 7,614,868	$ 6,988,940
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 798,438	$ 606,988
Total current liabilities	798,438	606,988
Noncurrent liabilities:		
Long-term convertible debt, net	596,663	595,138
Other long-term liabilities	163,056	128,824
Total liabilities	1,558,157	1,330,950
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 192,098,751 and 190,761,349 shares issued and outstanding, respectively	192	191
Additional paid-in capital	5,900,968	5,802,068
Company common stock held by the Nonqualified Deferred Compensation Plan	(11,291)	(11,227)
Accumulated other comprehensive income (loss)	(33,937)	61,653
Retained earnings (accumulated deficit)	200,779	(194,695)
Total stockholders' equity	6,056,711	5,657,990
Total liabilities and stockholders' equity	$ 7,614,868	$ 6,988,940

(1) December 31, 2024 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, 2024, filed with the SEC on February 24, 2025.

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

BIOMARIN PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
Three and Nine Months Ended September 30, 2025 and 2024
(In thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Shares of common stock, beginning balances [1]	192,002	190,356	190,761	188,598
Issuances under equity incentive plans	97	198	1,338	1,956
Shares of common stock, ending balances	192,099	190,554	192,099	190,554
Total stockholders' equity, beginning balances [1]	$ 6,027,113	$ 5,286,299	$ 5,657,990	$ 4,951,549
Common stock:				
Beginning balances [1]	192	190	191	189
Issuances under equity incentive plans, net of tax	—	1	1	2
Ending balances	192	191	192	191
Additional paid-in capital:				
Beginning balances [1]	5,851,637	5,696,701	5,802,068	5,611,562
Issuances under equity incentive plans, net of tax	(2,779)	(2,688)	(46,218)	(33,031)
Stock-based compensation	52,493	45,853	145,054	159,522
Change in Common stock held by the Nonqualified Deferred Compensation plan (NQDC)	(383)	44	64	1,857
Ending balances	5,900,968	5,739,910	5,900,968	5,739,910
Company common stock held by the NQDC:				
Beginning balances [1]	(11,674)	(11,673)	(11,227)	(9,860)
Common stock held by the NQDC	383	(44)	(64)	(1,857)
Ending balances	(11,291)	(11,717)	(11,291)	(11,717)
Accumulated other comprehensive income (loss):				
Beginning balances [1]	(44,565)	26,799	61,653	(28,788)
Other comprehensive income (loss)	10,628	(22,149)	(95,590)	33,438
Ending balances	(33,937)	4,650	(33,937)	4,650
Retained earnings (accumulated deficit)				
Beginning balances [1]	231,523	(425,718)	(194,695)	(621,554)
Net income (loss)	(30,744)	106,080	395,474	301,916
Ending balances	200,779	(319,638)	200,779	(319,638)
Total stockholders' equity, ending balances	$ 6,056,711	$ 5,413,396	$ 6,056,711	$ 5,413,396

(1) The beginning balances for the nine-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, 2024 filed with the SEC on February 24, 2025.

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

BIOMARIN PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
Nine Months Ended September 30, 2025 and 2024
(In thousands)
(unaudited)

	Nine Months Ended September 30,	
	2025	**2024**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net income	$ 395,474	$ 301,916
Adjustments to reconcile net income to net cash provided by operating activities:		
Depreciation and amortization	59,119	72,819
Non-cash interest expense	1,969	2,699
Accretion of discount on investments	(3,695)	(6,619)
Stock-based compensation	134,496	149,652
Gain on sale of nonfinancial assets	—	(10,000)
Impairment of assets and other noncash adjustments	2,967	19,889
Acquired in-process research & development expense	220,963	—
Deferred income taxes	48,125	13,709
Unrealized foreign exchange gain	244	(22,352)
Other	(1,704)	(1,254)
Changes in operating assets and liabilities:		
Accounts receivable, net	(107,378)	(130,456)
Inventory	(98,796)	(29,259)
Other current assets	(32,125)	(19,939)
Other assets	(32,679)	(31,839)
Accounts payable and accrued liabilities	119,478	68,019
Other long-term liabilities	21,892	10,229
Net cash provided by operating activities	728,350	387,214
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(62,316)	(65,894)
Maturities and sales of investments	294,547	478,436
Purchases of investments	(313,262)	(352,371)
Proceeds from sale of nonfinancial assets	—	10,000
Purchase of intangible assets	(5,569)	(11,225)
Acquisition, net of cash acquired	(285,193)	—
Other	—	1,141
Net cash provided by (used in) investing activities	(371,793)	60,087
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of awards under equity incentive plans	7,714	41,415
Taxes paid related to net share settlement of equity awards	(53,265)	(72,651)
Repayments of convertible debt	—	(494,987)
Other	—	(3,083)
Net cash used in financing activities	(45,551)	(529,306)
Effect of exchange rate changes on cash	(3,740)	2,326
NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	307,266	(79,679)
Cash and cash equivalents:		
Beginning of period	$ 942,842	$ 755,127
End of period	$ 1,250,108	$ 675,448
SUPPLEMENTAL CASH FLOW DISCLOSURES:		
Cash paid for interest	$ 3,572	$ 6,654
Cash paid for income taxes	$ 70,208	$ 36,088
SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON-CASH INVESTING AND FINANCING ACTIVITIES:		
Increase (decrease) in accounts payable and accrued liabilities related to fixed assets	$ 7,014	$ 8,457
Increase (decrease) in accounts payable and accrued liabilities related to intangible assets	$ (3,937)	$ (9,059)

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

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

(1) BUSINESS OVERVIEW AND SIGNIFICANT ACCOUNTING POLICIES

Nature of Operations

BioMarin Pharmaceutical Inc. (the Company or BioMarin) is a leading, global rare disease biotechnology company focused on delivering medicines for people living with genetically defined conditions. The San Rafael, California-based company, founded in 1997, has a proven track record of innovation, with eight commercial therapies and a strong clinical and preclinical pipeline. Using a distinctive approach to drug discovery and development, BioMarin seeks to unleash the full potential of genetic science by pursuing category-defining medicines that have a profound impact on patients.

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 Securities and Exchange Commission 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, 2024 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 nine months ended September 30, 2025 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2025 or any other period.

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 and has concluded that there were no subsequent events or transactions 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.

Significant Accounting Policies

Other than as discussed below, there have been no changes to the Company's significant accounting policies during the nine months ended September 30, 2025, 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, 2024.

Acquisitions

Acquisitions of businesses are accounted for using the acquisition method of accounting. The Company allocates the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based on their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and acquired in-process research and development (IPR&D) assets. Any excess of the purchase price over the estimated fair values of the net assets acquired is recognized as goodwill.

If it is determined that the net assets acquired do not meet the definition of a business combination under the acquisition method of accounting, the transaction is accounted for as an asset acquisition and no goodwill is recognized. Acquired IPR&D assets with no alternative future use under the acquisition method of accounting are charged to Research and Development in the Consolidated Statements of Income.

Recent Accounting Pronouncements

Other than as discussed below, 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 nine months ended September 30, 2025, 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, 2024, 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.

Income Taxes

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 the Company's related disclosures.

Income Statement Disaggregation

In November 2024, the FASB issued ASU 2024-03, *Income Statement - Reporting Comprehensive Income Topic 220, Expense Disaggregation Disclosures*. The guidance requires disclosure of additional information about specific expense categories in the notes to financial statements at interim and annual reporting periods. 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, 2026 and interim periods within fiscal years beginning after December 15, 2027. The Company is currently evaluating the effect of the update on the Company's related disclosures.

Internal-use Software

In September 2025, the FASB issued ASU 2025-06, *Intangibles - Goodwill and Other - Internal-Use Software Topic 350-40: Accounting for and Disclosure of Software Costs*. The guidance clarifies the accounting for costs related to internal-use software by removing all references to project stages and clarifying the threshold to be applied to begin capitalizing. The guidance also specifies that disclosure under Accounting Standards Codification Topic 360-10, *Property, Plant, and Equipment - Overall*, apply to capitalized software costs. The Company may apply the guidance using a prospective, retrospective or modified transition approach. The effective date for the update is for fiscal years beginning after December 15, 2027 and interim periods within fiscal years beginning after December 15, 2027. The Company is currently evaluating the effect of the update on the Company's related disclosures.

(2) FINANCIAL INSTRUMENTS

All marketable securities were classified as available-for-sale as of September 30, 2025 and December 31, 2024.

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

	September 30, 2025						
	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	$ 400,492	$ —	$ —	$ 400,492	$ 400,492	$ —	$ —
Level 2:							
Money market instruments	844,616	—	—	844,616	844,616	—	—
Corporate debt securities	474,892	3,520	(26)	478,386	—	181,605	296,781
U.S. government agency securities	202,584	991	(21)	203,554	5,000	45,912	152,642
Asset-backed securities	63,313	423	(8)	63,728	—	214	63,514
Subtotal	1,585,405	4,934	(55)	1,590,284	849,616	227,731	512,937
Total	$ 1,985,897	$ 4,934	$ (55)	$ 1,990,776	$ 1,250,108	$ 227,731	$ 512,937

	December 31, 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	$ 329,619	$ —	$ —	$ 329,619	$ 329,619	$ —	$ —
Level 2:							
Money market instruments	613,223	—	—	613,223	613,223	—	—
Corporate debt securities	503,202	2,410	(390)	505,222	—	168,104	337,118
Asset-backed securities	138,508	363	(344)	138,527	—	25,864	112,663
U.S. government agency securities	72,027	359	(33)	72,353	—	896	71,457
Subtotal	1,326,960	3,132	(767)	1,329,325	613,223	194,864	521,238
Total	$ 1,656,579	$ 3,132	$ (767)	$ 1,658,944	$ 942,842	$ 194,864	$ 521,238

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

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

As of September 30, 2025, 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.

(3) SUPPLEMENTAL FINANCIAL STATEMENTS INFORMATION

Inventory consisted of the following:

	September 30, 2025	December 31, 2024
Raw materials	$ 115,302	$ 154,341
Work-in-process	781,170	550,678
Finished goods	485,701	527,634
Total inventory	$ 1,382,173	$ 1,232,653

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

	September 30, 2025	December 31, 2024
Property, plant and equipment, gross	$ 1,987,843	$ 1,934,832
Accumulated depreciation	(949,656)	(891,791)
Total property, plant and equipment, net	$ 1,038,187	$ 1,043,041

Depreciation expense, net of amounts capitalized into inventory, for the three and nine months ended September 30, 2025 was $10.6 million and $35.3 million, respectively. Depreciation expense, net of amounts capitalized into inventory, for the three and nine months ended September 30, 2024 was $10.9 million and $35.0 million, respectively.

Intangible Assets, Net consisted of the following:

	September 30, 2025	December 31, 2024
Finite-lived intangible assets	$ 722,742	$ 721,110
Accumulated amortization	(489,630)	(465,832)
Net carrying value	$ 233,112	$ 255,278

Accounts Payable and Accrued Liabilities consisted of the following:

	September 30, 2025	December 31, 2024
Accounts payable and accrued operating expenses	$ 389,670	$ 235,403
Accrued compensation expense	177,755	202,513
Accrued rebates payable	140,049	120,835
Foreign currency exchange forward contracts	43,739	13,056
Accrued income taxes	22,606	12,567
Accrued royalties payable	6,945	7,923
Lease liability	7,578	7,574
Other	10,096	7,117
Total accounts payable and accrued liabilities	$ 798,438	$ 606,988

(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, 2024.

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 September 30, 2025 or

December 31, 2024. 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 September 30, 2025 or December 31, 2024.

Level 2 assets and liabilities that are remeasured using significant observable inputs consisted of the following, except for derivatives, which are discussed in Note 5 – *Derivative Instruments and Hedging Strategies*:

	September 30, 2025	December 31, 2024
Assets:		
Other current assets:		
NQDC Plan assets	$ 3,881	$ 2,928
Other assets:		
NQDC Plan assets	41,479	34,978
Restricted investments (1)	369	514
Total other assets	41,848	35,492
Total assets	$ 45,729	$ 38,420
Liabilities:		
Accounts payable and accrued liabilities:		
NQDC Plan liability	$ 3,881	$ 2,928
Other long-term liabilities:		
NQDC Plan liability	41,479	34,978
Total liabilities	$ 45,360	$ 37,906

(1) The restricted investments as of September 30, 2025 and December 31, 2024 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 nine months ended September 30, 2025.

(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 two years. 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	September 30, 2025	December 31, 2024
Derivatives designated as hedging instruments:		
Sell	$ 1,098,405	$ 1,371,816
Purchase	$ 258,318	$ 289,967
Derivatives not designated as hedging instruments:		
Sell	$ 346,944	$ 344,101
Purchase	$ 161,535	$ 63,617

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

Balance Sheet Location	September 30, 2025	December 31, 2024
Derivatives designated as hedging instruments:		
Derivative Assets		
Other current assets	$ 14,449	$ 60,192
Other assets	5,716	14,514
Subtotal	$ 20,165	$ 74,706
Derivative Liabilities		
Accounts payable and accrued liabilities	$ 42,918	$ 12,381
Other long-term liabilities	14,801	2,536
Subtotal	$ 57,719	$ 14,917
Derivatives not designated as hedging instruments:		
Derivative Assets		
Other current assets	$ 4,429	$ 4,934
Derivative Liabilities		
Accounts payable and accrued liabilities	$ 821	$ 675
Total Derivative Assets	$ 24,594	$ 79,640
Total Derivative Liabilities	$ 58,540	$ 15,592

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, 2024.

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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
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	$ (10,639)	$ 3,365	$ (157)	$ 5,617
Operating expenses	$ 2,500	$ 172	$ 1,408	$ 486
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	$ 945	$ (12,094)	$ (35,234)	$ 9,447

As of September 30, 2025, the Company expects to reclassify unrealized losses of $28.6 million from Accumulated Other Comprehensive Income (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*.

(6) DEBT

Convertible Notes

As of September 30, 2025, the Company had outstanding fixed-rate convertible notes for an undiscounted aggregate principal amount of $600.0 million. The convertible notes detailed below are senior subordinated convertible obligations, and interest is payable in arrears, semi-annually. The following table summarizes information regarding the Company's convertible debt:

	September 30, 2025	December 31, 2024
1.25% senior subordinated convertible notes due in May 2027 (the 2027 Notes)	$ 600,000	$ 600,000
Unamortized discount net of deferred offering costs	(3,337)	(4,862)
Total convertible debt, net	$ 596,663	$ 595,138
Fair value of fixed-rate convertible debt[1] :		
2027 Notes	$ 569,841	$ 558,894

(1) The fair value of the Company's fixed-rate convertible debt is based on open-market trades and 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, 2024.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Coupon interest expense	$ 1,875	$ 2,456	$ 5,625	$ 7,689
Accretion of discount on convertible notes	486	606	1,456	2,290
Amortization of debt issuance costs	27	67	80	364
Total interest expense on convertible debt	$ 2,388	$ 3,129	$ 7,161	$ 10,343

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, 2024 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.

	Three Months Ended September 30, 2025		
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Total
AOCI balance as of June 30, 2025	$ (47,794)	$ 3,229	$ (44,565)
Other comprehensive income (loss) before reclassifications	1,972	674	2,646
Less: gain (loss) reclassified from AOCI	(8,139)	—	(8,139)
Tax effect	—	(157)	(157)
Net current-period other comprehensive income (loss)	10,111	517	10,628
AOCI balance as of September 30, 2025	$ (37,683)	$ 3,746	$ (33,937)

	Three Months Ended September 30, 2024		
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Total
AOCI balance as of June 30, 2024	$ 28,187	$ (1,388)	$ 26,799
Other comprehensive income (loss) before reclassifications	(26,099)	9,752	(16,347)
Less: gain (loss) reclassified from AOCI	3,537	—	3,537
Tax effect	—	(2,265)	(2,265)
Net current-period other comprehensive income (loss)	(29,636)	7,487	(22,149)
AOCI balance as of September 30, 2024	$ (1,449)	$ 6,099	$ 4,650

	Nine Months Ended September 30, 2025		
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Total
AOCI balance as of December 31, 2024	$ 59,824	$ 1,829	$ 61,653
Other comprehensive income (loss) before reclassifications	(96,256)	2,513	(93,743)
Less: gain (loss) reclassified from AOCI	1,251	—	1,251
Tax effect	—	(596)	(596)
Net current-period other comprehensive income (loss)	(97,507)	1,917	(95,590)
AOCI balance as of September 30, 2025	$ (37,683)	$ 3,746	$ (33,937)

	Nine Months Ended September 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	34,312	6,826	41,138
Less: gain (loss) reclassified from AOCI	6,103	—	6,103
Tax effect	—	(1,597)	(1,597)
Net current-period other comprehensive income (loss)	28,209	5,229	33,438
AOCI balance as of September 30, 2024	$ (1,449)	$ 6,099	$ 4,650

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

(8) SEGMENT INFORMATION

The Company operates and is managed as one business segment which derives revenue from activities related to the development and commercialization of innovative therapies for people with serious and life-threatening rare diseases and medical conditions.

The Company's commercial organization is responsible for marketing its approved products worldwide. The Company's research and development (R&D) organization is responsible for research and discovery of new product candidates and supporting the development and registration efforts for potential new products. The Company's technical operations group is responsible for the development of manufacturing processes, supplying clinical drug product, and the manufacturing and distribution of the Company's commercial products. The Company is also supported by corporate staff functions.

The Company's Chief Executive Officer, as the Chief Operating Decision Maker (CODM), manages and allocates resources to the operations of the total company by assessing the overall level of resources available and how to best allocate them to support the Company's long-term company-wide strategic goals. In making this decision, the CODM uses consolidated financial information for the purposes of evaluating performance, allocating resources, setting incentive compensation targets and planning and forecasting for future periods.

The key measure of segment profit or loss used by the CODM to allocate resources and assess the Company's performance is its Consolidated Net Income, as reported on the Condensed Consolidated Statements of Comprehensive Income. The CODM's analysis includes a comparison to budgeted results. Segment assets provided to the CODM are consistent with those reported on the Condensed Consolidated Balance Sheets with particular emphasis on the Company's available liquidity including cash, cash equivalents, investments, accounts receivable and inventory.

The following table includes information about segment revenue, significant segment expenses, and segment measure of profitability:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Total revenues	$ 776,133	$ 745,740	$ 2,346,688	$ 2,106,602
Less:				
Cost of sales	140,085	188,457	441,733	444,096
R&D expenses				
Research and early pipeline	93,720	104,915	280,158	331,372
Later-stage clinical programs	251,650	10,078	279,074	17,792
Marketed products	64,108	69,908	170,285	224,511
SG&A expenses				
S&M expenses	131,327	108,945	355,958	351,103
G&A expenses	137,088	144,535	350,852	391,315
Other segment expense (income), net [1]	(11,101)	12,822	73,154	44,497
Net income (loss)	$ (30,744)	$ 106,080	$ 395,474	$ 301,916

(1) Other segment expense, net during the three months ended September 30, 2025 and 2024 include intangible asset amortization, interest income and expense, other income (expense) and income tax expense.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
VOXZOGO	$ 218,387	$ 189,785	$ 653,532	$ 526,597
VIMIZIM	182,755	178,158	586,525	548,694
NAGLAZYME	122,025	131,906	365,182	369,584
PALYNZIQ	108,778	90,634	307,985	254,634
ALDURAZYME	53,709	71,029	159,123	144,849
BRINEURA	47,953	37,005	137,007	121,361
KUVAN	24,025	28,115	76,195	92,576
ROCTAVIAN	3,180	7,235	22,889	15,516
Total net product revenues	760,812	733,867	2,308,438	2,073,811
Royalty and other revenues	15,321	11,873	38,250	32,791
Total revenues	$ 776,133	$ 745,740	$ 2,346,688	$ 2,106,602

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 distributed, marketed and sold exclusively by Sanofi worldwide.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
United States	$ 274,114	$ 235,515	$ 788,361	$ 655,963
Europe	195,184	191,017	667,900	607,755
Latin America	97,618	111,908	284,018	274,920
Rest of world	140,187	124,398	409,036	390,324
Total net product revenues marketed by the Company	707,103	662,838	2,149,315	1,928,962
ALDURAZYME net product revenues marketed by Sanofi	53,709	71,029	159,123	144,849
Total net product revenues	$ 760,812	$ 733,867	$ 2,308,438	$ 2,073,811

The following table illustrates the percentage of the Company's total Net Product Revenues attributed to the Company's largest customers for the periods presented.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Customer A	14 %	11 %	15 %	13 %
Customer B	12	10	11	10
Customer C	11	12	12	11
Total	37 %	33 %	38 %	34 %

Concentration Information

On a consolidated basis, one customer accounted for 23% of the Company's September 30, 2025 accounts receivable balance compared to December 31, 2024, when two customers accounted for 20% and 11% of the accounts receivable balance, respectively. As of September 30, 2025, and December 31, 2024, the accounts receivable balance for Sanofi included $135.7 million and $96.8 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, impact of new or increased tariffs and escalating trade tensions, regulatory uncertainty, 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 such 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 as of September 30, 2025 based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries.

(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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Cost of sales	$ 4,281	$ 5,092	$ 10,628	$ 12,110
Research and development	14,676	11,496	40,648	45,108
Selling, general and administrative	30,308	26,901	83,220	92,434
Total stock-based compensation expense	$ 49,265	$ 43,489	$ 134,496	$ 149,652

(10) EARNINGS (LOSS) PER COMMON SHARE

Potentially issuable shares of common stock include shares issuable upon the exercise of outstanding employee stock option awards, common stock issuable under the 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 (loss) per common share (common shares in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Numerator:				
Net income (loss), basic	$ (30,744)	$ 106,080	$ 395,474	$ 301,916
Add: Interest expense, net of tax, on the Company's convertible debt	—	1,832	5,499	5,495
Net income (loss), diluted	$ (30,744)	$ 107,912	$ 400,973	$ 307,411
Denominator:				
Weighted-average common shares outstanding, basic	192,032	190,429	191,639	189,806
Effect of dilutive securities:				
Common stock issuable under the Company's equity incentive plans	—	2,353	889	2,512
Common stock issuable under the Company's convertible debt	—	4,365	4,365	4,365
Weighted-average common shares outstanding, diluted	192,032	197,147	196,893	196,683
Earnings (loss) per common share, basic	$ (0.16)	$ 0.56	$ 2.06	$ 1.59
Earnings (loss) per common share, diluted	$ (0.16)	$ 0.55	$ 2.04	$ 1.56

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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Common stock issuable under the Company's equity incentive plans	12,390	9,414	11,501	9,255
Common stock issuable under the Company's convertible debt	4,365	—	—	—
Total number of potentially issuable shares	16,755	9,414	11,501	9,255

(11) 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 first disclosed in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, 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 and financial statements.

Contingent Payments

As of September 30, 2025, the Company was subject to contingent payments, primarily comprised of development, regulatory and commercial milestones. Those considered reasonably possible totaled $231.1 million, and the related contingencies are not expected to be met over the next 12 months.

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 September 30, 2025, such commitments were estimated at $549.0 million, of which $199.1 million is expected to be paid in 2025 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.

(12) ASSET ACQUISITION

On July 1, 2025, the Company completed its acquisition of Inozyme Pharma, Inc. (Inozyme), a publicly traded clinical-stage biopharmaceutical company dedicated to developing innovative therapeutics, for a total consideration of approximately $285 million, net of cash acquired. Upon closing, Inozyme became a wholly-owned subsidiary of BioMarin.

The Company accounted for this transaction as an asset acquisition since substantially all of the fair value of the gross assets acquired was concentrated in a single identifiable IPR&D asset, INZ-701 (now known as BMN 401). The Company recognized the acquired assets and assumed liabilities based on the consideration paid, inclusive of transaction costs, on a relative fair value basis. As the acquired IPR&D does not have an alternative future use, the Company recorded approximately $221 million in acquired IPR&D expense related to the INZ-701 IPR&D asset during the third quarter of 2025. The IPR&D charge is included in Research and Development on the Company's Condensed Consolidated Statements of Comprehensive Income (Loss).

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

The following summarizes the total consideration transferred and allocation to the assets acquired, liabilities assumed and acquired IPR&D:

Cash consideration for outstanding shares	$	260,424
Cash consideration for equity awards		9,950
Consideration paid to Inozyme		270,374
Repayment of Inozyme debt		49,095
Employee-related incentive payments		2,714
Transaction costs		6,950
Total consideration	$	329,133
Cash and cash equivalents	$	43,939
Other assets		10,779
Deferred tax assets		68,697
Other liabilities		(15,245)
Total identifiable assets acquired, net		108,170
Acquired IPR&D		220,963
Total assets and liabilities	$	329,133

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).

Overview

We are a leading, global rare disease biotechnology company focused on delivering medicines for people living with genetically defined conditions. Our San Rafael, California-based company, founded in 1997, has a proven track record of innovation, with eight commercial therapies and a strong clinical and preclinical pipeline. Using a distinctive approach to drug discovery and development, we seek to unleash the full potential of genetic science by pursuing category-defining medicines that have a profound impact on patients.

A summary of our commercial products, as of September 30, 2025, is provided below:

Commercial Products	Indication
VOXZOGO (vosoritide)	Achondroplasia
VIMIZIM (elosulfase alpha)	Mucopolysaccharidosis (MPS) IVA
NAGLAZYME (galsulfase)	MPS VI
PALYNZIQ (pegvaliase-pqpz)	Phenylketonuria (PKU)
ALDURAZYME (laronidase)	MPS I
BRINEURA (cerliponase alfa)	Neuronal ceroid lipofuscinosis type 2 (CLN2)
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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Total revenues	$ 776.1	$ 745.7	$ 2,346.7	$ 2,106.6
Cost of sales	$ 140.1	$ 188.5	$ 441.7	$ 444.1
Research and development (R&D)	$ 409.5	$ 184.9	$ 729.5	$ 573.7
Selling, general and administrative (SG&A)	$ 268.4	$ 253.5	$ 706.8	$ 742.4
Provision for income taxes	$ 4.4	$ 28.4	$ 114.2	$ 70.2
Net income (loss)	$ (30.7)	$ 106.1	$ 395.5	$ 301.9

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, impact of new or increased tariffs and escalating trade tensions, regulatory uncertainty, 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 2025. We believe that the combination of our internal research programs, partnerships and acquisitions of external assets will allow us to continue to develop and commercialize innovative therapies for patients with serious and life-threatening rare diseases and medical conditions. We periodically conduct strategic portfolio assessment of research and development programs to determine which we believe have the strongest combination of scientific merit, opportunity for commercial success and potential value creation for stockholders.

Based on such strategic portfolio assessments, certain programs that do not meet its threshold for further development and commercialization could be discontinued.

In October 2025, we announced our plan to pursue options to divest ROCTAVIAN, including exploring out-licensing opportunities. We plan to continue to make ROCTAVIAN commercially available in the U.S., Italy and Germany until next steps are finalized. We will continue to provide support and monitoring for people treated with ROCTAVIAN.

In September 2025, at the American Society for Bone and Mineral Research (ASBMR) Annual Meeting, we shared new data demonstrating improved spinal morphology – one of the factors that contributes to spinal stenosis, a leading cause of morbidity in achondroplasia – following treatment with VOXZOGO in children ages 5 and under. VOXZOGO is the only approved therapy with data showing a positive impact on spinal morphology, and these findings add to the extensive body of evidence supporting VOXZOGO's health benefits beyond improving growth.

In September 2025, we presented new data from the PALYNZIQ Phase 3 PEGASUS study in 12 to 17 year-olds demonstrating statistically significant blood phenylalanine (Phe) lowering compared to diet alone.

In August 2025, we announced that Phase 1 data in its healthy volunteers study with BMN 333, BioMarin's long-acting C-type natriuretic peptide (CNP), demonstrated area-under-the-curve (AUC) pharmacokinetic (PK) levels greater than three times the levels observed in other long-acting CNP studies, and no safety signals were noted.

In July 2025, we completed the acquisition of Inozyme Pharma, Inc. (Inozyme), a publicly traded clinical-stage biopharmaceutical company dedicated to developing innovative therapeutics. The acquisition is intended to strengthen our enzyme therapies portfolio by adding a late-stage enzyme replacement therapy, BMN 401 (formerly INZ-701), for the treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency. We accounted for this transaction as an asset acquisition since the lead asset, BMN 401, represents substantially all of the fair value of the gross assets acquired. See Note 12 to our accompanying Condensed Consolidated Financial Statements for additional information related to Inozyme acquisition.

See the risk factors described under "Business and Operational Risks" section in "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q.

Results of Operations

Net Product Revenues

Net Product Revenues consisted of the following:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
VOXZOGO	$ 218.4	$ 189.9	$ 28.5	$ 653.5	$ 526.6	$ 126.9
Enzyme Therapies:						
VIMIZIM	182.8	178.2	4.6	586.5	548.7	37.8
NAGLAZYME	122.0	131.9	(9.9)	365.2	369.6	(4.4)
PALYNZIQ	108.8	90.6	18.2	308.0	254.6	53.4
ALDURAZYME	53.7	71.0	(17.3)	159.1	144.8	14.3
BRINEURA	48.0	37.0	11.0	137.0	121.4	15.6
KUVAN	23.9	28.1	(4.2)	76.2	92.6	(16.4)
ROCTAVIAN	3.2	7.2	(4.0)	22.9	15.5	7.4
Total net product revenues	$ 760.8	$ 733.9	$ 26.9	$ 2,308.4	$ 2,073.8	$ 234.6

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 September 30, 2025 as compared to the three months ended September 30, 2024 was primarily attributed to the following:

- VOXZOGO and PALYNZIQ: higher sales volume from new patients initiating therapy across all regions.

These increases were partially offset by the following:

- ALDURAZYME: lower sales volume due to timing of order fulfillment to Sanofi; and
- NAGLAZYME: lower sales volume due to timing of orders in countries that place large government orders, primarily in Latin America.

The increase in Net Product Revenues for the nine months ended September 30, 2025 as compared to the nine months ended September 30, 2024 was primarily attributed to the following:

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

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 “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.” in “Risk Factors” included in Part II, Item 1A of this Quarterly Report for additional information on risks we face.

With respect to VOXZOGO, see also the risk factor “Our success depends on our ability to manage our growth and execute our corporate strategy.” in "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 September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Sales denominated in USD	$ 398.6	$ 378.9	$ 19.7	$ 1,160.6	$ 1,017.5	$ 143.1
Sales denominated in foreign currencies	362.2	355.0	7.2	1,147.8	1,056.3	91.5
Total net product revenues	$ 760.8	$ 733.9	$ 26.9	$ 2,308.4	$ 2,073.8	$ 234.6

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Unfavorable impact of foreign currency exchange rates on product sales denominated in currencies other than USD	$ (8.2)	$ (22.6)	$ 14.4	$ (28.9)	$ (75.2)	$ 46.3

The unfavorable impact for the three and nine months ended September 30, 2025 was primarily driven by weakening of the Argentine Peso, Brazilian Real and Mexican Peso, partially offset by strengthening of the Euro. The unfavorable impact for the three and nine months ended September 30, 2024 was primarily driven by weakening of Argentine Peso, Japanese Yen and Brazilian Real, partially offset by strengthening of the Euro.

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 September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Total revenues	$ 776.1	$ 745.7	$ 30.4	$ 2,346.7	$ 2,106.6	$ 240.1
Cost of sales	$ 140.1	$ 188.5	$ (48.4)	$ 441.7	$ 444.1	$ (2.4)
Gross margin	81.9 %	74.7 %	7.2 %	81.2 %	78.9 %	2.3 %

Cost of Sales decreased in the three months ended September 30, 2025 as compared to the three months ended September 30, 2024, primarily due to favorable product mix resulting from lower per-unit manufacturing costs and reduced ROCTAVIAN inventory reserves. Gross margin increased during the three and nine months ended September 30, 2025 as compared to the three and nine months ended September 30, 2024, primarily due to higher sales volume for products with higher margin in our Enzyme Therapies portfolio.

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.

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 expense consisted of the following:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Research and early pipeline	$ 93.7	$ 104.9	$ (11.2)	$ 280.1	$ 331.4	$ (51.3)
Later-stage clinical programs	251.7	10.1	241.6	279.1	17.8	261.3
Marketed products	64.1	69.9	(5.8)	170.3	224.5	(54.2)
Total R&D expense	$ 409.5	$ 184.9	$ 224.6	$ 729.5	$ 573.7	$ 155.8

The increase in R&D expense for the three and nine months ended September 30, 2025 as compared to the three and nine months ended September 30, 2024 was primarily due to acquired In-Process Research and Development (IPR&D) related to a phase 3 clinical program from Inozyme acquisition and increased spend related to the continued progression of VOXZOGO indication for hypochondroplasia. These increases were partially offset by lower spend on Research and early pipeline due to discontinued programs and lower spend on ROCTAVIAN.

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 expense consisted of the following:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
S&M	$ 131.3	$ 108.9	$ 22.4	$ 356.0	$ 351.1	$ 4.9
G&A	137.1	144.6	(7.5)	350.8	391.3	(40.5)
Total SG&A expense	$ 268.4	$ 253.5	$ 14.9	$ 706.8	$ 742.4	$ (35.6)

S&M expense consisted of the following:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Enzyme Therapies	$ 60.9	$ 52.2	$ 8.7	$ 165.5	$ 163.8	$ 1.7
VOXZOGO	46.1	28.0	18.1	123.5	90.4	33.1
ROCTAVIAN	8.3	17.0	(8.7)	22.1	65.0	(42.9)
Other	16.0	11.7	4.3	44.9	31.9	13.0
Total S&M expense	$ 131.3	$ 108.9	$ 22.4	$ 356.0	$ 351.1	$ 4.9

The increase in S&M expense for the three and nine months ended September 30, 2025 as compared to the three and nine months ended September 30, 2024 was primarily due to increased spending related to global expansion of VOXZOGO for achondroplasia and pre-launch activities of VOXZOGO for hypochondroplasia, partially offset by reduced activities related to ROCTAVIAN as we focused commercial efforts in the U.S., Germany and Italy to align with our updated ROCTAVIAN strategy announced during the second half of 2024.

The decrease in G&A expense for the three and nine months ended September 30, 2025 as compared to the three and nine months ended September 30, 2024 was primarily due to severance and restructuring costs incurred in 2024 associated with our portfolio strategy review and the associated organizational redesign efforts that did not occur in 2025. The decrease in G&A expense was partially offset by post-acquisition severance charges related to Inozyme in 2025 and incremental administrative costs related to ongoing support of business initiatives during the year.

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 September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Amortization of intangible assets	$ 4.8	$ 5.0	$ (0.2)	$ 14.5	$ 33.6	$ (19.1)
Gain on sale of nonfinancial assets	$ —	$ —	$ —	$ —	$ 10.0	$ (10.0)

The decrease in Amortization of Intangible Assets for the nine months ended September 30, 2025 as compared to the nine months ended September 30, 2024 was due to the increase in the estimated useful life of an intangible asset as a result of the

extension of a patent during the second half of 2024 and an intangible asset becoming fully amortized during the fourth quarter of 2024.

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 September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Interest income	$ 17.9	$ 18.1	$ (0.2)	$ 55.7	$ 57.2	$ (1.5)

The change in Interest Income for the three and nine months ended September 30, 2025 as compared to the three and nine months ended September 30, 2024 was relatively flat.

Interest Expense

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Interest expense	$ 2.6	$ 3.0	$ (0.4)	$ 8.1	$ 10.1	$ (2.0)

The change in Interest Expense for the three and nine months ended September 30, 2025 as compared to the three and nine months ended September 30, 2024 was relatively flat. See Note 6 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our debt.

Other Income, Net

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Other income, net	$ 5.1	$ 5.5	$ (0.4)	$ 8.0	$ 2.2	$ 5.8

The increase in Other Income, Net for the nine months ended September 30, 2025 as compared to the nine months ended September 30, 2024 was primarily due to proceeds from insurance related to damaged goods. The change in Other Income, Net for the three months ended September 30, 2025 as compared to the three months ended September 30, 2024 was relatively flat.

Provision for Income Taxes

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Provision for income taxes	$ 4.4	$ 28.4	$ (24.0)	$ 114.2	$ 70.2	$ 44.0

The decrease in Provision for Income Taxes for the three months ended September 30, 2025 as compared to the three months ended September 30, 2024 was primarily due to lower pre-tax income, partially offset by unfavorable tax impact of a non-deductible acquired IPR&D charge in 2025. The increase in Provision for Income Taxes for the nine months ended September 30, 2025 as compared to the nine months ended September 30, 2024 was primarily due to higher pre-tax income and tax expense related to expiration of unexercised options in 2025.

On July 4, 2025, the One Big Beautiful Bill (OBBB) Act, which includes a broad range of tax reform provisions, was signed into law in the United States. We have estimated the tax effects of the OBBB Act enacted during the quarter and included the related income tax benefit impact in our provision for income taxes. We will continue to evaluate the full impact of these legislative changes as additional guidance becomes available.

Financial Condition, Liquidity and Capital Resources

Our cash, cash equivalents, and investments as of September 30, 2025 and December 31, 2024 were as follows:

	September 30, 2025	December 31, 2024	Change
Cash and cash equivalents	$ 1,250.1	$ 942.8	$ 307.3
Short-term investments	227.7	194.9	32.8
Long-term investments	512.9	521.2	(8.3)
Total cash, cash equivalents and investments	$ 1,990.7	$ 1,658.9	$ 331.8

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, impact of new or increased tariffs and escalating trade tensions, regulatory uncertainty, 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 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:

	Nine Months Ended September 30,		
	2025	2024	Change
Net cash provided by operating activities	$ 728.4	$ 387.2	$ 341.2
Net cash provided by (used in) investing activities	$ (371.8)	$ 60.1	$ (431.9)
Net cash used in financing activities	$ (45.6)	$ (529.3)	$ 483.7

The increase in net cash provided by operating activities in the nine months ended September 30, 2025 compared to September 30, 2024 was primarily attributed to an increase in net income adjusted for non-cash items, and timing of payments to vendors and cash receipts from our customers.

The decrease in net cash provided by investing activities in the nine months ended September 30, 2025 compared to September 30, 2024 was primarily attributable to approximately $285 million net cash paid for acquisition of Inozyme and lower net maturities of available-for-sale securities. The decrease was partially offset by fewer purchases of fixed assets and intangible assets.

The decrease in net cash used in financing activities in the nine months ended September 30, 2025 compared to September 30, 2024 was primarily due to $495 million settlement of the 2024 Notes that matured in August 2024. The decrease was also attributable to lower proceeds from exercises of equity awards in 2025.

Financing

Our $600.0 million (undiscounted) of convertible debt as of September 30, 2025 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 September 30, 2025, our indebtedness consisted of 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 May 2027. 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, 2024.

In August 2024, we entered into an unsecured revolving credit facility providing for $600.0 million in revolving loan commitments. The credit facility is intended to finance ongoing working capital needs and for other general corporate purposes. The credit facility contains financial covenants including a maximum total net leverage ratio and a minimum interest coverage ratio. The credit facility matures in August 2029. As of September 30, 2025, there were no amounts outstanding under the credit facility and we were in compliance with all covenants.

Material Cash Requirements

Purchase Obligations

As of September 30, 2025, we had obligations of approximately $549.0 million, of which $199.1 million is expected to be paid in 2025. 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 September 30, 2025, we were subject to contingent payments considered reasonably possible of $231.1 million, and the related contingencies are not expected to be met over the next 12 months. See Note 11 to our accompanying Condensed Consolidated Financial Statements for additional discussion on our contingent obligations.

Our lease commitments and unrecognized tax benefits as of September 30, 2025 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, 2024.

See Note 11 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 nine months ended September 30, 2025, 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, 2024, filed with the SEC on February 24, 2025.

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 nine months ended September 30, 2025 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, 2024.

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 September 30, 2025.

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

Except as otherwise noted, 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.

In January 2025, we began deploying a new ERP at certain subsidiaries, replacing existing operating and financial systems. The new ERP implementation is scheduled to occur in phases through 2026, with post-implementation activities following thereafter. 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 have updated our internal control over financial reporting, as necessary, to accommodate related changes in our financial management processes resulting from this implementation. As the implementation and post-implementation activities take place, we will continue to 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

None.

Item 1A. Risk Factors

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

We have marked with an asterisk (*) those risk factors below that include a substantive change from or update to the risk factors included in our Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the SEC on February 24, 2025.

Business and Operational Risks

***Our success depends on our ability to manage our growth and execute our corporate strategy.**

In 2024, we announced our new corporate strategy focused on innovation, growth, and value commitment, which includes, among other things, the acceleration or discontinuation of certain programs, the expansion of our pipeline, updates to our commercial organizational model, and cost transformation. If we are unable to successfully execute our strategy, our business, financial condition and results of operations may be materially and adversely affected.

As part of the strategy, we have announced that we are advancing VOXZOGO for the treatment of conditions beyond achondroplasia, including hypochondroplasia, idiopathic short stature, Noonan syndrome, Turner syndrome, and SHOX deficiency. 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 addition, in July 2025 we acquired Inozyme Pharma, Inc. (Inozyme) to strengthen our Enzyme Therapies portfolio, adding a late-stage enzyme replacement therapy, BMN 401 (formerly INZ-701). 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. 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 corporate strategy will generate its expected benefits and the costs associated with implementing such strategy may be greater than anticipated. The execution of such strategy 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 execute on our corporate strategy or realize its expected benefits, then our business, operating results and financial condition may be materially and adversely affected.

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 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.

***We have in the past and may in the future pursue acquisitions of other companies or businesses, which could divert our management's attention, fail to achieve the anticipated benefits and/or expose us to other risks or difficulties.**

As part of our new corporate strategy, we have acquired, and we may continue to acquire, companies or businesses that we believe could complement, expand or enhance our product offerings. For example, in July 2025, we completed the acquisition of Inozyme to strengthen our Enzyme Therapies portfolio with BMN 401, which is currently being assessed for the treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) Deficiency. Our acquisition strategy may divert the attention of management and cause us to incur various expenses in identifying, investigating and pursuing suitable acquisitions, whether or not such acquisitions are consummated.

In addition, once an acquisition is closed, integrating our business practices and operations with the acquired business' so that we can fully realize the anticipated benefits of the acquisition could require us to devote significant management attention and resources. The success of current and future acquisitions will depend, in part, on our ability to realize the anticipated benefits from successfully combining our and the acquired businesses' operations. We may face risks or experience difficulties successfully integrating acquired businesses, such as Inozyme, with our operations. Such difficulties could result in the failure to achieve revenue that we anticipate, the loss of key employees that may be difficult to replace in the very competitive pharmaceutical field, the failure to harmonize both companies' corporate cultures, the disruption of each company's ongoing businesses or inconsistencies in standards, controls, procedures and policies that adversely affect our ability to maintain relationships with suppliers, collaboration partners, clinical trial investigators or managers of our clinical trials.

Acquisitions could also result in dilutive issuances of equity securities, the incurrence of debt, contingent liabilities, amortization expenses, impairment of goodwill and/or purchased long-lived assets, and restructuring charges, any of which could adversely affect our operating results and financial condition. In addition, acquired product candidates, such as BMN 401, may not result in regulatory approval, may not perform as expected, may not be successful, may require significantly greater resources and investments than originally anticipated or may not produce the revenues, earnings or business synergies that we anticipated. As a result, the anticipated benefits of an acquisition may not be realized fully within the expected timeframe or at all or may take longer to realize or cost more than expected, which could materially and adversely affect our business, financial condition, results of operations and growth prospects.

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. 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.

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 VOXZOGO, other companies are developing, and may in the future develop, products for treatment for achondroplasia that, if approved, could potentially compete with VOXZOGO even during the period of orphan drug exclusivity, for example by using an alternative formulation or a different delivery technology. As we commercialize our products, we have faced and may continue to face intense competition from other pharmaceutical companies, some of which may have more extensive resources and/or established relationships in the communities we seek to treat. 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.

We also face competition from generic versions of our products. For example, generic versions of KUVAN are available in several countries around the world, including in the U.S. and the European Union (EU), which has adversely affected and will continue to adversely affect our revenues from KUVAN. Competitors launching generic versions of our products independently establish the price of such products and determine the types of discounts or rebates they will offer parties that purchase or pay for the product. Generic competition often results in decreases in the net prices at which branded products can be sold. After any introduction of a generic product, a significant percentage of the prescriptions written for our branded products will likely be filled with the generic product. Certain U.S. state laws allow for, and in some instances in the absence of specific instructions from the prescribing physician mandate, the dispensing of generic products rather than branded products when a generic version is available. We expect that the approval and launch of generic versions of our products and the approval and launch of branded products that compete with our products will continue to have a negative impact and could have a material adverse effect on our sales of our products and on our business, financial condition, results of operations and growth prospects.

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. We expect that most families of patients will not be capable of paying for our treatments themselves. For most of our products, we expect patients to need treatment for extended periods, and for some products throughout the lifetimes of the patients. 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 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.

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.

***Changes in methods of treatment of disease or failure of our products to gain acceptance by patients or the medical community could negatively impact demand for our products and adversely affect revenues.**

Even if our product candidates are approved, if doctors were to 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.

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 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. Although administration of a gene therapy product like ROCTAVIAN is intended to correct an inborn genetic defect for at least several years, if the therapeutic effect of ROCTAVIAN decreases significantly or ceases entirely, it is uncertain whether redosing would be possible or effective. 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.

In addition, if we do not accurately forecast demand or manufacture products at levels in alignment with actual demand due to the failure of our products to gain acceptance by the patients or the medical community or other factors, then we may experience product shortages, pay a fee to contract manufacturers with whom we have non-cancellable capacity reservation agreements, or build excess inventory that may need to be written off. For example, if we have ROCTAVIAN inventory in amounts that exceed actual demand, then such excess inventory may need to be written off, or we may incur an impairment charge with respect to the facility where ROCTAVIAN is manufactured, all of which could adversely affect our operating results.

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. For example, in December 2023, we entered into a Cooperation Agreement with Elliott Investment Management L.P., Elliott Associates, L.P. and Elliott International, L.P., which expired in December 2024 pursuant to the terms of the agreement. 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. 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 approval from the U.S. Food and Drug Administration (FDA) for each product candidate that we intend to commercialize, and in the EU, we must obtain approval from the European Commission (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 April 26, 2023, the EC adopted a proposal for a new Directive and Regulation to revise the existing pharmaceutical legislation. While discussions are still ongoing as part of the legislative process, this proposal 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. Our 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.

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 authorities and members of the public, including competitors, 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 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 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. 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 began 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), expanded 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 and price increase 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 may 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 became applicable in January 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 EC and EU Member States cannot accept another marketing authorization (MA) application or accept an application to extend existing authorizations for similar medicinal products for the same indication and no 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, for example, "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 Biologics License Application (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.

Our products approved under BLAs in the U.S., Marketing Authorization Applications (MAAs) in the EU, or comparable regulatory approval applications in other countries, as well as our product candidates that may be approved in the future, could be reference products for biosimilar marketing applications. In the U.S., a follow-on biologic may be deemed interchangeable with, and automatically substitutable for, a reference product if its sponsor can 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. Even though the BPCIA establishes a period of 12 years of data exclusivity for reference products, 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.

The approval of biosimilar products referencing any of our products could have a material adverse impact on sales of our products and on our business, financial condition, results of operations and growth prospects due to increased competition and pricing pressures.

***Disruptions at the FDA, the EMA, other comparable regulatory authorities and other government agencies 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.**

Disruptions at regulatory authorities and government agencies due to changes in funding levels, government shutdowns, reorganization, reduction in force or statutory, regulatory and policy changes can affect their ability to hire and retain key personnel and carry out their normal functions that support our business. For example, the current U.S. administration recently implemented or proposed policies, including substantial reductions to the FDA’s workforce, that may affect the FDA’s review process and hinder the ability of the FDA to timely review and approve regulatory submissions for our product candidates. 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 the most recent shut down that began on October 1, 2025. 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 fail to obtain the capital necessary to fund our operations, our financial results and financial condition will be adversely affected and we will have to delay or terminate some or all of our product development programs.**

As of September 30, 2025, we had cash, cash equivalents and investments totaling $2.0 billion and debt obligations of $600.0 million (undiscounted), which consisted of our 1.25% senior subordinated convertible notes due in 2027 (the 2027 Notes). 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, protect, 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;
- our future plans for strategic investments and/or acquisitions; 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 September 30, 2025, we had $600.0 million (undiscounted) principal amount of indebtedness, all of which was outstanding under the 2027 Notes. In August 2024, we entered into an unsecured credit agreement (the 2024 Credit Agreement) with Citibank, N.A., as the administrative agent, and the other lenders party thereto, providing for $600.0 million in revolving loan commitments (the 2024 Credit Facility). The 2024 Credit Facility matures in August 2029. As of September 30, 2025, no amounts were outstanding under the 2024 Credit Facility. Our indebtedness may:

- limit our ability to borrow additional funds for working capital, capital expenditures, acquisitions or other general business purposes;

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

In addition, the 2024 Credit Facility contains, and any future indebtedness that we may incur may contain, financial and other restrictive covenants that limit our ability to operate our business, raise capital or make payments under our other indebtedness. If we fail to comply with these covenants or to make payments under our indebtedness when due, then we would be in default under that indebtedness, which could, in turn, result in that and our other indebtedness becoming immediately payable in full. If we default under the 2024 Credit Agreement, the outstanding borrowings thereunder could become immediately due and payable, the 2024 Credit Facility lenders could refuse to permit additional borrowings under the facility, or it could lead to defaults under agreements governing our current or future indebtedness, including the indenture governing the 2027 Notes. If we default under the 2027 Notes, the 2027 Notes could become immediately due and payable and it could lead to defaults under the 2024 Credit Agreement.

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 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. In addition, we also may borrow up to $600.0 million in revolving loans under the 2024 Credit Facility, which would be required to be repaid in cash at maturity in August 2029.

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, we lost potential VOXZOGO revenues in the past due to strong demand outpacing our projections and supply constraints. Although such supply constraints have since been resolved, we may lose potential revenues with respect to our products or experience adverse impacts on our development programs in the future if our manufacturing capabilities are unable to meet demand.

The development of commercially viable manufacturing processes typically is very difficult to achieve and is often very expensive and may require extended periods of time. Changes in manufacturing processes (including manufacturing cell lines), equipment or facilities (including moving manufacturing from one of our facilities to another one of our facilities or a third-party facility, or from a third-party facility to one of our facilities) may require us to complete clinical trials to receive regulatory approval of any manufacturing modifications.

Also, we may be required to demonstrate product comparability between a biological product made after a manufacturing change and the product made before implementation of the change through additional types of analytical and functional testing or may have to complete additional nonclinical or 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 and the conflicts in the Middle East;
- diminished protection of intellectual property in some countries outside of the U.S.;
- impact of new or increased tariffs, other trade protection measures (such as import or export licensing requirements), and escalating trade tensions;
- difficulty in staffing and managing international operations;
- differing labor regulations and business practices;
- parallel trade in our products, such as importation or our products, whether legally or illegally, from countries where our products are sold at lower prices into countries where the products are sold at higher prices;
- 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 but have, or are in the process, of being officially approved for national reimbursement in countries where patient numbers are sufficient for it to apply. 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 Japanese Yen, the Brazilian Real, the Argentine Peso, the Canadian Dollar, the Colombian 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 (as has been the case against the Euro in 2025), 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.

***Global trade issues and changes in and uncertainties with respect to trade policies and export regulations, including import and export license requirements, trade sanctions, tariffs and international trade disputes, could adversely impact our business and operations, and reduce the competitiveness of our products and services relative to local and global competitors.**

We operate in a global economy, and our business depends on a global supply chain for the development, manufacturing, and distribution of our pharmaceutical products, and for the advancement of our preclinical and clinical development programs. There is inherent risk, based on the complex relationships among the United States and the countries in which we conduct our business, that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations. The current international trade and regulatory environment is subject to significant ongoing uncertainty.

The ongoing trade tensions between the United States and other jurisdictions have resulted in multiple rounds of tariffs and anticipated tariffs affecting pharmaceuticals and pharmaceutical ingredients, including finished drug products, manufacturing equipment, and related supplies. Such tariffs may significantly increase our costs for certain products. The Bureau of Industry and Security, U.S. Department of Commerce, has initiated an investigation to determine whether pharmaceutical ingredients, including finished drug product, manufactured outside the United States pose a national security risk and should be subject to additional tariffs. Unlike consumer goods, pharmaceuticals face unique regulatory constraints that make rapid supply chain adjustments particularly difficult and costly. Since we conduct manufacturing and packaging operations in Ireland for certain of our products and also engage contract manufacturers around the world, the import of our products into the United States is subject to tariffs. The magnitude and impact of tariffs are uncertain and are subject to a variety of factors, including the effective date and duration of additional tariffs, changes in the amount, scope and nature of tariffs in the future, any retaliatory tariffs that other countries may impose in response to tariffs levied by the United States and any mitigating actions that may become available. Trade restrictions and export regulations, or increases in tariffs and additional taxes, including any retaliatory measures, could negatively impact demand, increase our supply chain complexity and our manufacturing costs, decrease margins, reduce the competitiveness of our products, or restrict our ability to sell products, provide services or purchase necessary equipment and supplies, any or all of which could have a material and adverse effect on our business, results of operations, or financial condition. In addition, the dynamic and unpredictable tariff and trade landscape creates substantial uncertainty and significant planning challenges for our operations and complicates our long-term investment decisions regarding manufacturing facilities, supply chain optimization, and research and development locations.

Current or future tariffs could result in increased research and development expenses, including with respect to increased costs associated with APIs, raw materials, laboratory equipment and research materials and components. Trade restrictions

affecting the import of materials necessary for clinical trials could result in delays to our development timelines. Increased development costs and extended development timelines could place us at a competitive disadvantage compared to companies operating in regions with more favorable trade relationships and could reduce investor confidence and negatively impact our business, results of operations, financial condition and growth prospects.

The complexity of announced or future tariffs may also increase the risk that we or our customers or suppliers may be subject to civil or criminal enforcement actions in the United States or foreign jurisdictions related to compliance with trade regulations. Foreign governments may also adopt non-tariff measures, such as procurement preferences or informal disincentives to engage with, purchase from or invest in U.S. entities, which may limit our ability to compete internationally and attract non-U.S. investment, employees, customers and suppliers. Foreign governments may also take other retaliatory actions against U.S. entities, such as decreased intellectual property protection, increased enforcement actions, or delays in regulatory approvals, which may result in heightened international legal and operational risks. In the event foreign governments impose tariffs or other retaliatory measures against U.S. entities, our ability to pass increased costs to customers in such foreign jurisdictions may be limited by the structure of pharmaceutical pricing and reimbursement systems and how any such tariffs were implemented in such countries. In addition, the United States and other governments have imposed and may continue to impose additional sanctions, such as trade restrictions or trade barriers, which could restrict us from doing business directly or indirectly in or with certain countries or parties and may impose additional costs and complexity to our business.

Trade disputes, tariffs, restrictions and other political tensions between the United States and other countries may also exacerbate unfavorable macroeconomic conditions including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns. The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this report.

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.

In addition, we have pursued, and may in the future pursue, litigation, administrative challenges or other types of proceedings to protect our patents and intellectual property rights. Such proceedings are often protracted and expensive, and have an unpredictable outcome.

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. Additionally, if our employees, consultants or contractors use generative artificial intelligence (AI) technologies to develop our proprietary technology and compounds, it may impact our ability to obtain or successfully defend certain intellectual property rights. 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 have in the past and may in the future 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 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;
- the impact of new or increased tariffs and escalating trade tensions;
- 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 public announcements by us or our competitors; 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. 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. We have experienced changes to our management team starting with the appointment of Alexander Hardy as our new President and Chief Executive Officer in December 2023. Since then, Cristin Hubbard was appointed as our new Executive Vice President and Chief Commercial Officer in May 2024, Dr. Greg Friberg was appointed as our new Executive Vice President and Chief Research & Development Officer in September 2024, and Dr. James Sabry was appointed as our new Executive Vice President and Chief Business Officer in October 2024. The recent changes in our management team, organizational structure, and corporate strategy could cause retention and morale concerns among current employees, and may create 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. Moreover, during the second and third quarters of 2024, we announced reductions in our global workforce by an aggregate of approximately 395 employees. As a result, we could face employee attrition beyond our intended reductions in force and adverse effects on employee morale, diversion of management attention, and adverse effects to our reputation as an employer, which could make it more difficult for us to hire employees in the future. 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.

***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, legislation referred to as the One Big Beautiful Bill (OBBB) Act enacted in 2025, along with prior U.S. federal tax reform legislation, enacted many significant changes to the U.S. taxation of business entities, including, among other changes, the imposition of minimum taxes and excise taxes under certain circumstances, changes to the taxation of income

derived from international operations, changes in the deduction and amortization of research and development expenditures, and limitations on the deductibility of business interest. For example, for tax years beginning after December 31, 2024, the OBBB Act restores the tax deductibility of domestic research and development expenses in the year incurred, which expenses had been required under prior legislation to be capitalized and subsequently amortized over five years. The OBBB Act did not change the tax treatment of expenses incurred in research and development activities conducted outside the United States, which expenses continue to be required to be capitalized and amortized over 15 years. We are evaluating the potential impacts this and other changes under the OBBB Act may have on our business. Future guidance from the Internal Revenue Service and other tax authorities with respect to any legislation may affect us, and certain aspects of such legislation could be repealed or modified in future legislation or sunset in future years. 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 the implementation of proposals, commonly referred to as “BEPS 2.0”, which, 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. A number of countries in which we conduct business have enacted, or are in the process of enacting, core elements of Pillar Two rules. Based on the above thresholds, we currently expect that we are within the scope of the Pillar Two rules. However we do not currently expect such rules to have a material adverse impact on our effective tax rate. 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, 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 first disclosed in our Annual Report on Form 10-K for the year ended December 31, 2023, 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.

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 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 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.

***We, and the third parties with whom we work, are subject to stringent and evolving U.S. and foreign laws, regulations and rules, contractual obligations, industry standards, policies and other obligations related to data privacy and security. Actual or perceived failure to comply with such obligations by us or the third parties with whom we work could lead to regulatory investigations or actions, litigation, fines and penalties, disruptions of our business operations, reputational harm, loss of revenue or profits, and other adverse business consequences.**

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, sensitive third-party data, business plans, transactions, financial information and medical information collected by our patient access management team (collectively, sensitive data). Our data processing activities subject us to certain data privacy

and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to data privacy and security.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). For example, HIPAA, as amended by HITECH, imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Additionally, numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018 (CCPA) requires businesses to provide specific disclosures in privacy notices and honor requests of California residents to exercise certain privacy rights. The CCPA provides for fines for intentional violations and allows private litigants affected by certain data breaches to recover significant statutory damages. Although some U.S. comprehensive privacy laws exempt some data processed in the context of clinical trials, these laws may increase compliance costs and potential liability with respect to other personal data we may maintain about California residents. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more jurisdictions to pass similar laws in the future.

Outside the United States, an increasing number of laws, regulations, and industry standards may govern data privacy and security. For example, the European Union's General Data Protection Regulation (EU GDPR), United Kingdom's GDPR (UK GDPR) (collectively, the GDPR), Brazil's General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or LGPD) (Law No. 13,709/2018), and China's Personal Information Protection Law (PIPL) impose strict requirements for processing personal data. For example, under the GDPR, companies may face temporary or definitive bans on data processing and other corrective actions; fines of up to 20 million Euros under the EU GDPR / 17.5 million pounds sterling under the UK GDPR or 4% of annual global revenue, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests.

In addition, we may be unable to transfer personal data from Europe and other jurisdictions to the United States or other countries due to data localization requirements or limitations on cross-border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area (EEA) and the UK have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it believes are inadequate. Other jurisdictions have adopted may adopt similarly stringent data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us to transfer personal data from the EEA, the UK, or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including by limiting our ability to conduct clinical trial activities in Europe and elsewhere, the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data to recipients outside Europe for allegedly violating the GDPR's cross-border data transfer limitations. Additionally, companies that transfer personal data to recipients outside of the EEA and/or UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators individual litigants and activist groups.

Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restriction on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered individuals (i.e., individuals and entities located in or controlled by individuals or entities located in those jurisdictions) that may impact certain business activities such as vendor engagements, sale or sharing of data, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to transfer data in connection with certain transactions or agreements.

We are subject to new laws governing the privacy of consumer health data, including reproductive, sexual orientation, and gender identity privacy rights that provide consumers certain rights with respect to their health data and create a private right of action to allow individuals to sue for violations.

Our employees and personnel, as well as third parties with whom we work, use, or may in the future use, generative AI technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If these factors limit or otherwise impair our effective use of generative AI, it could make our business less efficient and result in competitive disadvantages.

In addition to data privacy and security laws, we are contractually subject to industry standards adopted by industry groups and may become subject to additional such obligations in the future. We are also bound by other contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We publish privacy policies, marketing materials, and other statements, such as statements related to compliance with certain certifications or self-regulatory principles, regarding data privacy and security. Regulators in the United States are increasingly scrutinizing these statements, and if these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences.

Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources and may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process personal data on our behalf.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties with whom we work fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans or restrictions on processing personal data; and orders to destroy or not use personal data. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to loss of customers; inability to process personal data or to operate in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or 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 adopted and came into force in December 2024. These rules apply to products placed on the market or put into service as of December 9, 2025, and makes it easier for patients to claim damages for defective products, for example by alleviating their burden of proof.

We, and the third parties with whom we work, 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.

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 or interruptions associated with the implementation of new or upgraded technology systems or with maintenance or adequate support of existing systems could disrupt or reduce, and has in the past disrupted or reduced, the efficiency of our operations and expose us to greater risk of security breaches. Cybersecurity incidents resulting in the failure of our ERP 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. In particular, we have implemented certain modules of a new global ERP system and will continue to implement other components of the new system, which has replaced, and will continue to replace, legacy 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.

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. 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 unauthorized activity such as malware, hacking, business email compromise, phishing and other social engineering attacks (including deep fakes, which may be increasingly more difficult to identify as fake), ransomware or other cyberattacks. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments. Remote work has also increased risks to our information technology systems and data, as our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations.

Future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

While we have implemented measures to protect our information systems and data stored in those systems and those of the third parties with whom we work, our efforts may not be successful. It may also be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Our efforts to do so may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses, and disruptions of our business. We rely on third parties to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, cloud-based infrastructure, data center facilities, encryption and authentication technology, employee email, clinical trial functions, manufacturing partners, and other functions. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If the third parties with whom we work experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if the third parties with whom we work fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award.

We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties with whom we work). We may not, however, detect and remediate all such vulnerabilities including on a timely basis. Further, we may experience delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident.

Any of the previously identified or similar threats could cause a security incident or other interruption that could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties with whom we work. We have experienced and may continue to experience security 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.

In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position.

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, wildfire, or other catastrophic disaster could cause damage to our facilities 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 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 and the conflicts in the Middle East, tariffs and escalating trade tensions, 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 September 30, 2025, none of our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated the contracts, instructions or written plans for the purchase or sale of BioMarin securities.

Item 6. Exhibits

Exhibit Number	Description
2.1	Agreement and Plan of Merger, dated as of May 16, 2025, by and among Inozyme Pharma, Inc., BioMarin Pharmaceutical Inc., and Incline Merger Sub, Inc., previously filed with the SEC on May 16, 2025 as Exhibit 2.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
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 March 3, 2025 as Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
31.1*	Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
32.1*+	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.
101.INS	XBRL Instance Document - 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 With Embedded Linkbase Documents
101.CAL*	Inline XBRL Taxonomy Extension Calculation Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase
101.LAB*	Inline XBRL Taxonomy Extension Labels Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Link Document
104	XBRL tags for the cover page from the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2025, are embedded within the Inline XBRL document.

* Filed herewith

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

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

(i) Condensed Consolidated Balance Sheets as of September 30, 2025 and December 31, 2024, (ii) Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2025 and 2024, (iii) Condensed Consolidated Statement of Stockholders' Equity for the three and nine months ended September 30, 2025 and 2024, (iv) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2025 and 2024, 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: October 28, 2025

By /S/ BRIAN R. MUELLER

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

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: October 28, 2025

/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: October 28, 2025

/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 September 30, 2025, 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: October 28, 2025

/s/ BRIAN R. MUELLER

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

Date: October 28, 2025

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.