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, 2026

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: 193,694,734 shares of common stock, par value $0.001, outstanding as of October 26, 2026.

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®, GALAFOLD®, KUVAN®, NAGLAZYME®, PALYNZIQ®, POMBILITI® + OPFOLDA®, 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. You should not place undue reliance on these types of forward-looking statements, which speak only as of the date that they were made. These forward-looking statements are based on the beliefs and assumptions of our management based on information currently available to management and should be considered in connection with any written or oral forward-looking statements that we may issue in the future as well as other cautionary statements we have made and may make. 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, 2025, which was filed with the Securities and Exchange Commission (the SEC) on February 26, 2026. You should carefully consider that information before you make an investment decision.

Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this Quarterly Report on Form 10-Q may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Except as required by law, we do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Quarterly Report on Form 10-Q to reflect later events or circumstances or the occurrence of unanticipated events.

The 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.

Risks Related to the Amicus Acquisition

- We may not realize the anticipated benefits from the Amicus Acquisition or accurately forecast the future performance of the combined company.
- We have incurred and expect to incur material expenses related to the Amicus Acquisition.
- We may not realize the anticipated cost savings from the Amicus Acquisition.

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.
- We have incurred in the past and may in the future incur 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.

Manufacturing Risks

- If we fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.
- If we are unable to successfully develop and maintain manufacturing processes for our product candidates to produce sufficient quantities at acceptable costs, we may be unable to support a clinical trial or be forced to terminate a program, or if we are unable to produce sufficient quantities of our products at acceptable costs, we may be unable to meet commercial demand, lose potential revenue, have reduced margins or be forced to terminate a program.
- Supply interruptions may disrupt our inventory levels and the availability of our products and product candidates and cause delays in obtaining regulatory approval for our product candidates, or harm our business by reducing our revenues.

Risks Related to International Operations

- We conduct a significant amount of our operations and generate a significant percentage of our sales outside of the U.S., which subjects us to additional business risks that could adversely affect our revenues and results of operations.
- A significant portion of our international sales are made based on special access programs, and changes to these programs could adversely affect our product sales and revenues in these countries.
- Our international operations pose currency risks, which may adversely affect our operating results and net income.

Intellectual Property Risks

- If we are unable to protect our intellectual property, we may not be able to compete effectively or preserve our market shares.
- Competitors and other third parties may have developed intellectual property that could limit our ability to market and commercialize our products and product candidates, if approved.

BIOMARIN PHARMACEUTICAL INC.

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)

Three and Nine Months Ended September 30, 2026 and 2025

(In thousands, except per share amounts)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	**2025**	**2026**	**2025**
REVENUES:				
Net product revenues	$ 1,013,080	$ 760,812	$ 2,757,551	$ 2,308,438
Royalty and other revenues	11,566	15,321	23,011	38,250
Total revenues	1,024,646	776,133	2,780,562	2,346,688
OPERATING EXPENSES:				
Cost of sales	205,416	140,085	603,210	441,733
Research and development	180,525	188,515	566,273	508,554
Acquired in-process research and development (IPR&D)	287,685	220,963	287,685	220,963
Selling, general and administrative	333,643	268,415	987,482	706,810
Intangible asset amortization	99,458	4,847	177,433	14,540
Total operating expenses	1,106,727	822,825	2,622,083	1,892,600
INCOME (LOSS) FROM OPERATIONS	(82,081)	(46,692)	158,479	454,088
Interest income	4,630	17,854	37,670	55,694
Interest expense	(55,628)	(2,579)	(133,881)	(8,121)
Other income, net	1,310	5,093	8,550	7,972
INCOME (LOSS) BEFORE INCOME TAXES	(131,769)	(26,324)	70,818	509,633
Provision for (benefit from) income taxes	(8,938)	4,420	43,360	114,159
NET INCOME (LOSS)	$ (122,831)	$ (30,744)	$ 27,458	$ 395,474
EARNINGS (LOSS) PER SHARE, BASIC	$ (0.63)	$ (0.16)	$ 0.14	$ 2.06
EARNINGS (LOSS) PER SHARE, DILUTED	$ (0.63)	$ (0.16)	$ 0.14	$ 2.04
Weighted average common shares outstanding, basic	193,590	192,032	193,171	191,639
Weighted average common shares outstanding, diluted	193,590	192,032	195,214	196,893
COMPREHENSIVE INCOME (LOSS)	$ (118,059)	$ (20,116)	$ 36,920	$ 299,884

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

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

September 30, 2026 and December 31, 2025

(In thousands, except share amounts)

	September 30, 2026	December 31, 2025
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 736,007	$ 1,311,679
Short-term investments	—	248,930
Accounts receivable, net	1,076,355	908,214
Inventory	1,796,069	1,298,883
Other current assets	312,205	185,784
Total current assets	3,920,636	3,953,490
Noncurrent assets:		
Long-term investments	—	492,242
Property, plant and equipment, net	980,081	952,508
Intangible assets, net	4,777,247	213,837
Goodwill	666,367	196,199
Deferred tax assets	897,787	1,508,697
Other assets	327,113	277,049
Total assets	$ 11,569,231	$ 7,594,022
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 946,107	$ 759,031
Current portion of long-term debt, net	658,717	—
Total current liabilities	1,604,824	759,031
Noncurrent liabilities:		
Long-term debt, net	3,514,974	597,176
Other long-term liabilities	193,401	150,816
Total liabilities	5,313,199	1,507,023
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 193,676,831 and 192,300,101 shares issued and outstanding, respectively	194	192
Additional paid-in capital	6,089,036	5,956,582
Company common stock held by the Nonqualified Deferred Compensation Plan	(10,851)	(10,508)
Accumulated other comprehensive loss	(4,011)	(13,473)
Retained earnings	181,664	154,206
Total stockholders' equity	6,256,032	6,086,999
Total liabilities and stockholders' equity	$ 11,569,231	$ 7,594,022

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, 2026 and 2025
(In thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Shares of common stock, beginning balances [1]	193,535	192,002	192,300	190,761
Issuances under equity incentive plans	142	97	1,377	1,338
Shares of common stock, ending balances	193,677	192,099	193,677	192,099
Total stockholders' equity, beginning balances [1]	$ 6,321,691	$ 6,027,113	$ 6,086,999	$ 5,657,990
Common stock:				
Beginning balances [1]	194	192	192	191
Issuances under equity incentive plans, net of tax	—	—	2	1
Ending balances	194	192	194	192
Additional paid-in capital:				
Beginning balances [1]	6,037,020	5,851,637	5,956,582	5,802,068
Issuances under equity incentive plans, net of tax	(4,790)	(2,779)	(37,730)	(46,218)
Stock-based compensation	57,189	52,493	169,841	145,054
Change in Common stock held by the Nonqualified Deferred Compensation plan (NQDC)	(383)	(383)	343	64
Ending balances	6,089,036	5,900,968	6,089,036	5,900,968
Company common stock held by the NQDC:				
Beginning balances [1]	(11,234)	(11,674)	(10,508)	(11,227)
Common stock held by the NQDC	383	383	(343)	(64)
Ending balances	(10,851)	(11,291)	(10,851)	(11,291)
Accumulated other comprehensive loss:				
Beginning balances [1]	(8,783)	(44,565)	(13,473)	61,653
Other comprehensive income (loss)	4,772	10,628	9,462	(95,590)
Ending balances	(4,011)	(33,937)	(4,011)	(33,937)
Retained earnings (accumulated deficit)				
Beginning balances [1]	304,495	231,523	154,206	(194,695)
Net income (loss)	(122,831)	(30,744)	27,458	395,474
Ending balances	181,664	200,779	181,664	200,779
Total stockholders' equity, ending balances	$ 6,256,032	$ 6,056,711	$ 6,256,032	$ 6,056,711

(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, 2025 filed with the SEC on February 26, 2026.

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, 2026 and 2025
(In thousands)
(unaudited)

	Nine Months Ended September 30,	
	2026	**2025**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net income	$ 27,458	$ 395,474
Adjustments to reconcile net income to net cash provided by operating activities:		
Depreciation and amortization	244,514	59,119
Non-cash interest expense	30,548	1,969
Stock-based compensation	172,684	134,496
Impairment of assets	15,203	2,967
Acquired IPR&D expense	282,258	220,963
Deferred income taxes	(27,490)	48,125
Unrealized foreign exchange losses (gains)	(8,380)	244
Other	(7,091)	(5,399)
Changes in operating assets and liabilities, net of effects of business acquired:		
Accounts receivable, net	(60,435)	(107,378)
Inventory	(1,621)	(98,796)
Other current assets	(54,369)	(32,125)
Other assets	12,859	(32,679)
Accounts payable and accrued liabilities	(52,675)	119,478
Other long-term liabilities	3,511	21,892
Net cash provided by operating activities	576,974	728,350
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(78,032)	(62,316)
Maturities and sales of investments	767,277	294,547
Purchases of investments	(25,792)	(313,262)
Business combination, net of cash acquired	(5,067,630)	—
Purchases of IPR&D, net of cash acquired	(277,881)	(285,193)
Other investing activities, net	(1,191)	(5,569)
Net cash used in investing activities	(4,683,249)	(371,793)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of awards under equity incentive plans	6,655	7,714
Taxes paid related to net share settlement of equity awards	(43,330)	(53,265)
Proceeds from borrowings	3,650,000	—
Payments of debt issuance costs	(65,605)	—
Repayments of borrowings	(15,000)	—
Net cash provided by (used in) financing activities	3,532,720	(45,551)
Effect of exchange rate changes on cash	(2,117)	(3,740)
NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	(575,672)	307,266
Cash and cash equivalents:		
Beginning of period	$ 1,311,679	$ 942,842
End of period	$ 736,007	$ 1,250,108
SUPPLEMENTAL CASH FLOW DISCLOSURES:		
Cash paid for interest	$ 66,048	$ 3,572
Cash paid for income taxes	$ 74,378	$ 70,208
SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON-CASH INVESTING AND FINANCING ACTIVITIES:		
Accounts payable and accrued liabilities related to fixed assets	$ 9,623	$ 6,040

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. Founded in 1997, the San Rafael, California-based company has a proven track record of innovation, with nine 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 unaudited Condensed Consolidated Financial Statements have been prepared pursuant to U.S. generally accepted accounting principles (U.S. GAAP) and the rules and regulations of the SEC for Quarterly Reports on Form 10-Q and do not include all of the information and note disclosures required by U.S. GAAP for complete financial statements, although management believes that the disclosures herein are adequate to ensure that the information presented is not misleading. The Condensed Consolidated Financial Statements should therefore be read in conjunction with the Consolidated Financial Statements and Notes thereto for the fiscal year ended December 31, 2025 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, 2026 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2026 or any other period.

Acquired IPR&D was previously included in Research and development (R&D) and is now presented separately in the Condensed Consolidated Statements of Comprehensive Income (Loss). The Company believes this presentation provides users of the financial statements with a better understanding of upfront and development milestone payments incurred to acquire IPR&D projects. Prior-period amounts have been reclassified to conform to the current presentation, resulting in a reclassification of $221.0 million from R&D expense to Acquired IPR&D for the three and nine months ended September 30, 2025. This reclassification had no impact on total operating expenses, net income (loss) or earnings (loss) per share.

On April 27, 2026, the Company completed the acquisition of Amicus Therapeutics, Inc. (Amicus). The results of Amicus' operations, along with the preliminary estimated fair values of assets acquired and liabilities assumed in the acquisition, have been included in the Condensed Consolidated Financial Statements since the closing of the acquisition on April 27, 2026. Refer to Note 2 – *Acquisitions* for additional details related to the Amicus acquisition.

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, except for the transaction disclosed in Note 15 to these Condensed Consolidated Financial Statements.

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.

Significant Accounting Policies

There were no changes to the Company's significant accounting policies during the nine months ended September 30, 2026, other than the updates as described below. The Company's significant accounting policies are disclosed in Note 1 – *Business Overview and Significant Accounting Policies* to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2025.

Foreign Currency

While the functional currency of the Company and certain subsidiaries continues to be the U.S. Dollar (USD), the functional currency of certain acquired foreign subsidiaries is their respective local currency.

For subsidiaries whose functional currency is the USD, monetary assets and liabilities denominated in foreign currencies are remeasured using period-end exchange rates and non-monetary assets and liabilities are remeasured using historical exchange rates. Foreign currency transaction gains and losses resulting from the remeasurement are recognized in Other Income (Expense), Net in the Consolidated Statements of Income.

For subsidiaries whose functional currency is their local currency, assets and liabilities are translated into USD using exchange rates in effect at the balance sheet date and income and expense items are translated using average foreign exchange rates for the period. Adjustments resulting from the translation of the financial statements of these certain foreign subsidiaries into USD are recorded in Accumulated Other Comprehensive Income (Loss), a separate component of stockholders' equity. Foreign currency transaction gains and losses arising from transactions denominated in a currency other than the subsidiary's functional currency are recognized in Other Income (Expense), Net in the Consolidated Statements of Income.

Acquired IPR&D

Acquired IPR&D expenses are recorded when incurred and reflect costs of externally-developed IPR&D projects acquired directly in a transaction other than a business combination that do not have an alternative future use. These expenses include upfront and pre-commercialization milestone payments and the costs of rights to IPR&D projects. Subsequent development costs related to these projects are recognized as Research and development expense in the Consolidated Statements of Income as incurred.

Recent Accounting Pronouncements

There have been no new accounting pronouncements adopted by the Company or new accounting pronouncements issued by the Financial Accounting Standards Board (FASB) during the nine months ended September 30, 2026, 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, 2025, that the Company believes are of significance or potential significance to the Company.

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

(2) ACQUISITIONS

Amicus Acquisition

On April 27, 2026, the Company completed the acquisition of Amicus, a publicly traded, global, biotechnology company. Under the terms of the merger agreement, the Company acquired all outstanding shares of Amicus common stock, and upon closing, Amicus became a wholly-owned subsidiary of BioMarin. The acquisition is expected to strengthen the Company's commercial portfolio by adding two new treatments to its existing portfolio of medicines that target lysosomal storage diseases: GALAFOLD (migalastat), the first oral treatment for Fabry disease, and POMBILITI (cipaglucosidase alfa-atga) + OPFOLDA (miglustat), a two-component therapy for Pompe disease. In connection with the acquisition, the Company also received U.S. rights to BMN 820 (also known as DMX-200), a potential first-in-class oral CCR2 inhibitor for focal segmental glomerulosclerosis (FSGS), a rare fatal kidney disease in Phase 3 development.

The total purchase consideration for the acquisition of Amicus was $5.3 billion, which consisted of cash consideration paid to former Amicus shareholders, cash payments related to the settlement of equity awards and stock options, the settlement of Amicus' outstanding debt, and certain seller transaction costs paid by the Company on behalf of Amicus.

The transaction was financed through a combination of cash on hand and non-convertible debt financing. In connection with the acquisition, the Company issued $850.0 million in aggregate principal amount of 5.5% senior unsecured notes in February 2026, and obtained senior secured term loan facilities for $2.8 billion in aggregate principal in April 2026. Refer to Note 7 – *Debt* for additional details.

The transaction has been accounted for as a business combination using the acquisition method of accounting, which requires, among other things, that the assets acquired and liabilities assumed be recognized at their fair value on the acquisition date. The Company recorded acquisition-related costs of $32.8 million in Selling, General and Administrative (SG&A) on the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) during the nine months ended September 30, 2026.

Total consideration transferred for the acquisition of Amicus is summarized as follows:

Cash paid to Amicus' shareholders	$	4,563,463
Cash settlement towards Amicus' RSUs and PSUs		142,636
Cash settlement towards pre-combination portion of stock options [(1)]		62,946
Transaction expenses settled by the Company on behalf of Amicus		121,596
Amicus debt repayment by the Company on behalf of Amicus [(2)]		432,920
Total consideration	$	5,323,561

(1) Cash settlement towards the stock options consists of $51.9 million paid for vested stock options and $11.0 million paid for the pre-combination portion of unvested stock options that were accelerated as part of the acquisition. The fair value of the unvested stock options attributable to the post-combination period of $13.0 million was recorded within SG&A on the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) during the second quarter of 2026.

(2) Includes cash settlement related to repayment of outstanding debt, prepayment penalty and accrued interest.

The following table summarizes the preliminary purchase price allocation of the assets acquired and liabilities assumed as of April 27, 2026, adjusted as of September 30, 2026:

	Amounts Recognized as of Acquisition Date (as adjusted)[(1)]
Cash and cash equivalents	$ 255,931
Accounts receivable[(2)]	123,912
Inventory	469,700
Other current assets	55,052
Property, plant and equipment	25,923
Intangible assets	4,750,000

Goodwill	469,786
Other assets	59,219
Accounts payable and accrued liabilities	(203,549)
Deferred tax liabilities	(577,589)
Other long-term liabilities	(104,824)
Total assets and liabilities	**$ 5,323,561**

(1) Includes measurement period adjustments primarily related to income tax during the third quarter of 2026 that increased deferred tax liabilities and goodwill by approximately $10.6 million.

(2) The Company expects to collect all accounts receivable based on information available as of the acquisition date.

The purchase price allocation presented above is preliminary as of September 30, 2026 and remains subject to revision during the measurement period, which extends up to one year from the acquisition date. The preliminary estimates of the fair values assigned to assets acquired and liabilities assumed, including identifiable intangible assets, deferred income taxes, and other acquired assets and assumed liabilities, are based on information available as of the filing date. As the Company obtains additional information and completes its valuation analyses, it may record adjustments to these preliminary amounts, with a corresponding adjustment to goodwill.

Intangible Assets

The fair values of the commercialized pharmaceutical products and IPR&D were determined using the multi-period excess earnings method. This approach determines fair value based on cash flow projections which are discounted to present value using a risk-adjusted rate of return. The discount rate used to determine the fair values of commercialized pharmaceutical products and IPR&D were 12.0% and 12.5%, respectively. These fair value measurements were based on significant inputs not observable in the market and thus represent Level 3 fair value measurements.

The following table presents details of the purchased intangible assets acquired:

	Estimated Useful Life (in Years)	Fair Value
Commercialized pharmaceutical product – GALAFOLD	11	$ 3,000,000
Commercialized pharmaceutical product – POMBILITI + OPFOLDA	12	1,450,000
IPR&D - BMN 820	Indefinite	300,000
Total intangible assets		$ 4,750,000

The amortization expense related to acquired intangible assets for the three and nine months ended September 30, 2026 was $95.0 million and $164.0 million, respectively.

Inventory

The fair value of inventory acquired includes approximately $228.0 million step-up for GALAFOLD and POMBILITI + OPFOLDA. The fair value was primarily determined based on the estimated selling price of finished goods less the cost to complete the manufacturing process and selling effort. The amortization of the inventory step-up is recognized as a component of Cost of Sales on the Company's Consolidated Statements of Comprehensive Income (Loss) as the inventory is sold. The amortization expense related to inventory step-up for the three and nine months ended September 30, 2026 was $17.5 million and $29.2 million, respectively.

Leases

The Company recorded lease liabilities of approximately $53.8 million, which represent the present value of remaining lease payments over the respective lease terms, with a corresponding right-of-use assets of approximately $46.1 million, which represent the estimated fair value based on current market rental rates. As of September 30, 2026, the right-of-use assets are presented in Other assets on the Company's Condensed Consolidated Balance Sheets. As of September 30, 2026, the current portion of the lease liabilities is presented in Accounts Payable and Accrued Liabilities whereas the non-current portion is presented in Other Long-term Liabilities on the Company's Condensed Consolidated Balance Sheets.

Goodwill

The recognized goodwill is primarily attributed to a deferred tax liability associated with the acquired intangible assets and inventory step-up. Goodwill recognized for Amicus is not deductible for tax purposes.

Results of Operations and Pro forma Financial Information

The following table presents revenues and net earnings for Amicus since the acquisition date of April 27, 2026 through September 30, 2026:

	Amount
Total revenue	$ 336,422
Net loss	$ (239,383)

The unaudited pro forma results of operations for BioMarin as if the acquisition had occurred on January 1, 2025 are presented in the table below:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Total revenue	$ 1,024,646	$ 945,194	$ 3,009,554	$ 2,795,686
Net loss	$ (55,110)	$ (136,132)	$ (256,017)	$ (57,533)

These unaudited pro forma results were based on estimates and assumptions that the Company believes are reasonable and are presented for informational purposes only. The unaudited pro forma financial information is not indicative of the results of operations that would have been achieved had the acquisition and related financing occurred at the beginning of fiscal year 2025. The unaudited pro forma information includes adjustments such as inventory step-up, amortization for intangible assets acquired, stock-based compensation expense, interest expense for acquisition financing, transaction costs and related income tax effects.

The significant nonrecurring adjustments reflected in the unaudited pro forma information above include the impact of (i) transaction costs of $47.1 million, (ii) post-combination stock-based compensation expense of $13.0 million, and (iii) commitment fee of $22.8 million relating to bridge loan commitment, all of which have been recognized as if incurred on the assumed acquisition date of January 1, 2025.

Alesta Acquisition

On August 31, 2026, the Company completed its acquisition of Alesta Therapeutics, B.V. (Alesta), a Dutch private limited liability company, committed to developing novel oral small-molecule therapies for underserved diseases, for a total consideration of $303.4 million. The terms of the acquisition also provide for regulatory and sales milestone payments of up to $215 million. Upon closing, Alesta 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, BMN 440 (formerly ALE1). BMN 440 is an orally active, small molecule for the potential treatment of hypophosphatasia (HPP), a rare genetic bone disease caused by mutations in the ALPL gene. 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 $282.3 million in acquired IPR&D expense related to BMN 440 IPR&D asset during the third quarter of 2026. The IPR&D charge is included in Acquired In-process Research and Development on the Company's Condensed Consolidated Statements of Income.

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

Cash consideration for outstanding shares	$ 261,950
Cash consideration for equity awards	25,782
Consideration paid to Alesta	287,732
Transaction expenses settled by the Company on behalf of Alesta	8,256
Transaction expenses directly incurred by BioMarin	7,453
Total consideration	$ 303,441
Cash and cash equivalents	$ 22,158
Other assets	2,250
Other liabilities	(3,225)
Total identifiable assets acquired, net	21,183
Acquired IPR&D	282,258
Total assets and liabilities	$ 303,441

(3) FINANCIAL INSTRUMENTS

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of the dates presented. There were no marketable securities as of September 30, 2026 and all marketable securities as of December 31, 2025 were classified as available-for-sale.

	September 30, 2026				
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents
Level 1:					
Cash	$ 594,217	$ —	$ —	$ 594,217	$ 594,217
Level 2:					
Money market instruments	141,790	—	—	141,790	141,790
Total	$ 736,007	$ —	$ —	$ 736,007	$ 736,007

	December 31, 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	$ 415,760	$ —	$ —	$ 415,760	$ 415,760	$ —	$ —
Level 2:							
Money market instruments	895,919	—	—	895,919	895,919	—	—
Corporate debt securities	472,572	3,286	(3)	475,855	—	189,566	286,289
U.S. government agency securities	206,018	1,197	(1)	207,214	—	59,115	148,099
Asset-backed securities	57,687	420	(4)	58,103	—	249	57,854
Subtotal	1,632,196	4,903	(8)	1,637,091	895,919	248,930	492,242
Total	$ 2,047,956	$ 4,903	$ (8)	$ 2,052,851	$ 1,311,679	$ 248,930	$ 492,242

(1) The Company's short-term marketable securities as of December 31, 2025 matured in one year or less.

(2) The Company's long-term marketable securities as of December 31, 2025 matured between one and five years.

(4) SUPPLEMENTAL FINANCIAL STATEMENTS INFORMATION

Inventory consisted of the following:

	September 30, 2026	December 31, 2025
Raw materials	$ 272,552	$ 106,510
Work-in-process	1,234,970	801,061
Finished goods	288,547	391,312
Total inventory	$ 1,796,069	$ 1,298,883

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

	September 30, 2026	December 31, 2025
Property, plant and equipment, gross	$ 2,111,011	$ 2,026,813
Accumulated depreciation	(1,130,930)	(1,074,305)
Total property, plant and equipment, net	$ 980,081	$ 952,508

Depreciation expense, net of amounts capitalized into inventory, for the three and nine months ended September 30, 2026 was $10.2 million and $28.6 million, respectively. 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.

Intangible Assets, Net consisted of the following:

	September 30, 2026	December 31, 2025
Finite-lived intangible assets	$ 5,174,157	$ 723,966
Indefinite-lived intangible assets	300,000	—
Gross intangible assets:	5,474,157	723,966
Accumulated amortization	(696,910)	(510,129)
Net carrying value	$ 4,777,247	$ 213,837

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Accounts Payable and Accrued Liabilities consisted of the following:

	September 30, 2026	December 31, 2025
Accrued compensation expense	$ 246,436	$ 219,422
Accrued operating expenses	216,219	282,096
Accrued rebates payable	210,645	166,925
Accounts payable	130,717	30,672
Accrued Interest	37,480	907
Foreign currency exchange forward contracts	34,433	31,007
Accrued income taxes	18,918	3,667
Lease liabilities	16,519	8,685
Accrued royalties payable	15,768	7,968
Value added taxes payable	9,331	2,388
Other	9,641	5,294
Total accounts payable and accrued liabilities	$ 946,107	$ 759,031

(5) 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, 2025.

Other than the Company's debt obligations disclosed in Note 7 – *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, 2026 or December 31, 2025. Other than assets acquired from the Amicus acquisition discussed in Note 2 - *Acquisitions*, the Company had no financial assets or liabilities that are remeasured on a recurring or non-recurring basis using unobservable inputs that reflect estimates and assumptions (Level 3) as of September 30, 2026 or December 31, 2025.

Level 2 assets and liabilities that are remeasured using significant observable inputs consisted of the following, except for derivatives, which are discussed in Note 6 – *Derivative Instruments and Hedging Strategies* and the Term Loan A Facility, which is discussed in Note 7 - *Debt*:

	September 30, 2026	December 31, 2025
Assets:		
Other current assets:		
NQDC Plan assets	$ 5,004	$ 3,765
Other assets:		
NQDC Plan assets	44,994	41,689
Total assets	$ 49,998	$ 45,454
Liabilities:		
Accounts payable and accrued liabilities:		
NQDC Plan liability	$ 5,004	$ 3,765
Other long-term liabilities:		
NQDC Plan liability	44,994	41,689
Total liabilities	$ 49,998	$ 45,454

There were no transfers between levels during the three and nine months ended September 30, 2026.

(6) 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 USD, primarily the Euro. Certain of these forward contracts are designated as cash flow hedges and typically 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 typically 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, 2026	December 31, 2025
Derivatives designated as hedging instruments:		
Sell	$ 1,748,167	$ 1,573,184
Purchase	$ 353,048	$ 385,499
Derivatives not designated as hedging instruments:		
Sell	$ 475,311	$ 356,285
Purchase	$ 154,138	$ 36,798

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, 2026	December 31, 2025
Derivatives designated as hedging instruments:		
Derivative Assets		
Other current assets	$ 33,522	$ 17,585
Other assets	11,420	5,591
Subtotal	$ 44,942	$ 23,176
Derivative Liabilities		
Accounts payable and accrued liabilities	$ 33,127	$ 30,134
Other long-term liabilities	7,308	9,905
Subtotal	$ 40,435	$ 40,039
Derivatives not designated as hedging instruments:		
Derivative Assets		
Other current assets	$ 9,725	$ 2,479
Derivative Liabilities		
Accounts payable and accrued liabilities	$ 1,306	$ 873
Total Derivative Assets	$ 54,667	$ 25,655
Total Derivative Liabilities	$ 41,741	$ 40,912

For additional discussion of fair value measurements, see Note 5 - *Fair Value Measurements.*

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Derivatives Designated as Cash Flow Hedging Instruments	**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**		**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**	
Net product revenues	$ 100	$ (10,639)	$ (18,987)	$ (157)
Operating expenses	$ 379	$ 2,500	$ 6,600	$ 1,408
Derivatives Not Designated as Hedging Instruments	**Gains (Losses) Recognized in Earnings**		**Gains (Losses) Recognized in Earnings**	
Other income, net	$ (1,526)	$ 945	$ (2,545)	$ (35,234)

As of September 30, 2026, amounts expected to be reclassified from Accumulated Other Comprehensive Income (AOCI) to earnings over the next twelve months as the forecasted revenues and operating expense transactions occur were not material. For additional discussion of balances in AOCI see Note 8 – *Accumulated Other Comprehensive Income*.

(7) DEBT

As of September 30, 2026, the Company had outstanding fixed-rate notes and variable-rate term loans for an undiscounted aggregate principal amount of $4.2 billion. The debt detailed below incurs interest to be paid periodically in arrears. The following table summarizes information regarding the Company's debt:

	September 30, 2026	December 31, 2025
1.25% senior subordinated convertible notes due in May 2027 (the 2027 Notes)[1]	$ 600,000	$ 600,000
5.5% senior unsecured notes due in February 2034 (the 2034 Notes)	850,000	—
Term Loan A	790,000	—
Term Loan B	1,995,000	—
Total debt	4,235,000	600,000
Less: Unamortized discount and deferred offering costs	(61,309)	(2,824)
Total debt, net	$ 4,173,691	$ 597,176

Fair value of debt[2] :	**September 30, 2026**
2027 Notes	$ 585,884
2034 Notes	792,872
Term Loan A	789,013
Term Loan B	1,999,988
Total fair value of debt	$ 4,167,757

(1) For additional information related to the Company's 2027 Notes, 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, 2025.

(2) The fair value of the Company's debt instruments are based on open-market trades (Level 1 of the fair value hierarchy), except for the Term Loan A Facility, whose fair value was based on observable market inputs for comparable debt instruments (Level 2 of 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, 2025.

Interest expense related to the Company's debt and bridge loan commitment consisted of the following:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Coupon interest expense	$ 52,683	$ 1,875	$ 102,654	$ 5,625
Bridge commitment fee	—	—	22,813	—
Accretion of discount on convertible notes and term loans	2,063	486	4,120	1,456
Amortization of debt issuance costs	487	27	1,264	80
Total interest expense on debt	$ 55,233	$ 2,388	$ 130,851	$ 7,161

Term Loans and Revolving Credit Facility

In connection with the acquisition of Amicus in April 2026, the Company entered into a senior secured credit agreement (the 2026 Credit Agreement) consisting of an $800.0 million senior secured term loan A facility (Term Loan A Facility), a $2.0 billion senior secured term loan B facility (Term Loan B Facility, and, together with the Term Loan A Facility, the Term Facilities), and a $600.0 million senior secured revolving credit facility (the 2026 Revolving Facility). The proceeds from the Term Facilities were used to finance a portion of the acquisition consideration and related transaction costs. The Company incurred $46.2 million of issuance costs, which were deferred and will be amortized over the life of the Term Facilities and recorded as interest expense.

Borrowings under the Term Facilities and the 2026 Revolving Facility bear interest at variable rates based on Secured Overnight Financing Rate (SOFR) or an alternate base rate, plus an applicable margin as specified in the 2026 Credit Agreement. Interest on SOFR borrowings is payable at the end of each applicable interest period and, for interest periods greater than three months, at least quarterly.

The Term Loan A Facility matures on April 27, 2031 and requires quarterly principal repayments equal to 1.25% of the original principal amount, with the remaining outstanding principal balance due at maturity. The Term Loan B Facility matures on April 27, 2033 and requires quarterly principal repayments equal to 0.25% of the original principal amount, with the remaining outstanding principal balance due at maturity.

The 2026 Revolving Facility matures on April 27, 2031 and there were no borrowings outstanding under the 2026 Revolving Facility as of September 30, 2026. The Company had the full $600.0 million of borrowing capacity available under the facility, subject to compliance with the terms of the 2026 Credit Agreement. The 2026 Credit Agreement contains customary affirmative and negative covenants, including, among others, covenants that restrict the Company's ability to incur additional indebtedness, create liens, make investments, pay dividends or make other restricted payments, dispose of assets, and enter into transactions with affiliates, in each case subject to exceptions set forth therein.

2034 Notes

In February 2026, the Company issued $850.0 million in aggregate principal amount of 5.5% senior unsecured notes with a maturity date of February 15, 2034, and the proceeds from the issuance were deposited into an escrow account and subsequently used to fund the Amicus acquisition. The 2034 Notes bear interest at the rate of 5.5% per annum. Interest is payable semi-annually with cash in arrears on February 15 and August 15 of each year, beginning August 15, 2026. Net proceeds from the offering were $832.3 million. The Company incurred $17.7 million of issuance costs, which were deferred and will be amortized over the life of the 2034 Notes and recorded as interest expense.

The indenture governing the Notes contains customary covenants that, among other things, restrict, with certain exceptions, the ability of the Company and its subsidiaries to incur additional debt, pay dividends, make certain other restricted payments, incur debt secured by liens, dispose of assets, engage in consolidations and mergers or sell or transfer all or substantially all of its assets. The offer and sale of the 2034 Notes have not been registered under the Securities Act or any state securities laws.

Bridge Commitment

In December 2025, the Company entered into a debt financing commitment letter and related fee letter with certain lenders, pursuant to which the lenders committed to provide the Company with debt financing up to approximately $3.7 billion (the Bridge Commitment) in the form of a 364-day senior secured bridge loan facility (Bridge Facility), the proceeds of which would be available for the acquisition of Amicus. In connection with the issuance of the 2034 Notes, the Bridge Commitment was reduced to $2.8 billion. Upon entry into the senior secured term loan facility in April 2026, the remaining Bridge Commitment was reduced to zero. Refer to Note 2 – *Acquisitions* for additional details related to the Amicus acquisition.

(8) 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, 2026		
	Unrealized Gains (Losses) on Cash Flow Hedges	Cumulative Translation Adjustment	Total
AOCI balance as of June 30, 2026	$ (746)	$ (8,037)	$ (8,783)
Other comprehensive income (loss) before reclassifications	5,601	(350)	5,251
Reclassification of (gains) losses from AOCI	(479)	—	(479)
Tax effect	—	—	—
Net current-period other comprehensive income (loss)	5,122	(350)	4,772
AOCI balance as of September 30, 2026	$ 4,376	$ (8,387)	$ (4,011)

	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
Reclassification of (gains) losses 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)

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	Nine Months Ended September 30, 2026			
	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Cumulative Translation Adjustment	Total
AOCI balance as of December 31, 2025	$ (17,244)	$ 3,771	$ —	$ (13,473)
Other comprehensive income (loss) before reclassifications	9,233	(4,895)	(8,387)	(4,049)
Reclassification of (gains) losses from AOCI	12,387	—	—	12,387
Tax effect	—	1,124	—	1,124
Net current-period other comprehensive income (loss)	21,620	(3,771)	(8,387)	9,462
AOCI balance as of September 30, 2026	$ 4,376	$ —	$ (8,387)	$ (4,011)

	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)
Reclassification of (gains) losses 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)

For additional discussion of reclassifications from AOCI see Note 6 – *Derivative Instruments and Hedging Strategies*.

(9) 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 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 (Loss). 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,	
	2026	2025	2026	2025
Total revenues	$ 1,024,646	$ 776,133	$ 2,780,562	$ 2,346,688
Less:				
Cost of sales	205,416	140,085	603,210	441,733
R&D expenses				
Research and early pipeline	87,026	93,720	259,386	280,158
Later-stage clinical programs	23,656	30,687	110,128	58,111
Marketed products	69,843	64,108	196,759	170,285
Acquired IPR&D	287,685	220,963	287,685	220,963
SG&A expenses				
S&M expenses	148,916	131,327	416,029	355,958
G&A expenses	184,727	137,088	571,453	350,852
Other segment expense, net [1]	140,208	(11,101)	308,454	73,154
Net income (loss)	$ (122,831)	$ (30,744)	$ 27,458	$ 395,474

(1) Other segment expense, net during the three and nine months ended September 30, 2026 and 2025 includes intangible asset amortization, interest income and expense, other income, net and income tax expense.

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
VOXZOGO	$ 245,014	$ 218,387	$ 717,422	$ 653,532
Metabolic Conditions (formerly Enzyme Therapies):				
VIMIZIM	215,796	182,755	620,371	586,525
NAGLAZYME	107,824	122,025	373,153	365,182
PALYNZIQ	111,035	108,778	335,760	307,985
GALAFOLD[1]	151,831	—	257,579	—
BRINEURA	52,712	47,953	151,186	137,007
ALDURAZYME	56,598	53,709	136,879	159,123
POMBILITI + OPFOLDA[1]	48,585	—	78,843	—
KUVAN	22,947	24,025	71,271	76,195
ROCTAVIAN	738	3,180	15,087	22,889
Total net product revenues	1,013,080	760,812	2,757,551	2,308,438
Royalty and other revenues	11,566	15,321	23,011	38,250
Total revenues	$ 1,024,646	$ 776,133	$ 2,780,562	$ 2,346,688

(1) The Company commenced recognizing revenue related to GALAFOLD and POMBILITI + OPFOLDA on April 27, 2026 following its acquisition of Amicus. Refer to Note 2 - *Acquisitions* for additional information.

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,	
	2026	2025	2026	2025
United States	$ 352,049	$ 274,114	$ 966,067	$ 788,361
Europe	364,567	195,184	880,901	667,900
Latin America	92,313	97,618	304,394	284,018
Rest of world	147,553	140,187	469,310	409,036
Total net product revenues marketed by the Company	956,482	707,103	2,620,672	2,149,315
ALDURAZYME net product revenues marketed by Sanofi	56,598	53,709	136,879	159,123
Total net product revenues	$ 1,013,080	$ 760,812	$ 2,757,551	$ 2,308,438

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,	
	2026	2025	2026	2025
Customer A	12 %	12 %	12 %	11 %
Customer B	12	14	13	15
Customer C	10	11	11	12
Total	34 %	37 %	36 %	38 %

Concentration Information

On a consolidated basis, one customer accounted for 20% of the Company's September 30, 2026 accounts receivable balance compared to two customers who accounted for 20% and 10%, respectively, of the accounts receivable balance as of December 31, 2025. As of September 30, 2026, and December 31, 2025, the accounts receivable balance for Sanofi included $170.9 million and $148.0 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, wars and military conflicts, 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, 2026 based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. The Company will continue to monitor these conditions and will attempt to adjust its business processes, as appropriate, to mitigate macroeconomic risks to its business.

(10) STOCK-BASED COMPENSATION

The Company has stockholder-approved equity incentive plans that provide for the granting of 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 (Loss) for all stock-based compensation arrangements was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Cost of sales	$ 3,992	$ 4,281	$ 12,588	$ 10,628
Research and development	13,535	14,676	41,084	40,648
Selling, general and administrative	36,005	30,308	119,012	83,220
Total stock-based compensation expense	$ 53,532	$ 49,265	$ 172,684	$ 134,496

(11) RESTRUCTURING

Corporate Initiatives

In connection with the Company's plan to simplify its organizational design and strategic initiatives, the Company committed to plans in the first quarter of 2026 to reduce its global workforce. Restructuring charges associated with the reorganization and strategic initiatives comprised of cash expenditures for severance and other employee termination benefits. These reductions are expected to be substantially complete by the end of 2026.

In connection with the Company's corporate initiatives, the Company committed to a plan in the third quarter of 2026 to convert an office space to a laboratory, resulting in an asset impairment charge relating to building improvements.

Amicus

In April 2026, the Company committed to a restructuring plan related to the integration of Amicus. This restructuring plan is intended to realize certain targeted synergies following the acquisition, primarily through reductions in overlapping personnel costs within the general and administrative functions. Restructuring charges associated with Amicus integration comprised of cash expenditures for severance and other employee termination benefits. These reductions are expected to be completed in phases through April 2027.

Restructuring charges and adjustments were included in SG&A in the Condensed Consolidated Statements of Comprehensive Income (Loss). Restructuring charges consisted of the following:

	Three Months Ended September 30, 2026	Nine Months Ended September 30, 2026
Corporate initiatives - Severance	$ 4,057	$ 15,266
Corporate initiatives - Asset impairment	15,707	15,707
Amicus - Severance	29,478	82,618
	$ 49,242	$ 113,591

The following unpaid balance as of September 30, 2026 was recorded to Accounts Payable and Accrued Liabilities on the Condensed Consolidated Balance Sheets:

Balance as of December 31, 2025	$ 3,180
Additions and adjustments	97,884
Payments	(53,325)
Balance as of September 30, 2026	$ 47,739

(12) INCOME TAXES

The effective tax rates were 6.8% and (16.8)% for the three months ended September 30, 2026 and 2025, respectively, and 61.2% and 22.4% for the nine months ended September 30, 2026 and 2025, respectively. The effective tax rates for all periods reflected pre-tax losses resulting from nondeductible acquired IPR&D charges. The change in the effective tax rate for the three and nine months ended September 30, 2026 was primarily driven by the nondeductible acquired IPR&D charge having a greater impact on the estimated annual effective tax rate due to lower pre-tax income, which primarily reflected interest expense and amortization of acquired intangible assets related to the Amicus acquisition.

(13) 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,	
	2026	2025	2026	2025
Numerator:				
Net income (loss), basic	$ (122,831)	$ (30,744)	$ 27,458	$ 395,474
Add: Interest expense, net of tax, on the Company's convertible debt	—	—	—	5,499
Net income (loss), diluted	$ (122,831)	$ (30,744)	$ 27,458	$ 400,973
Denominator:				
Weighted-average common shares outstanding, basic	193,590	192,032	193,171	191,639
Effect of dilutive securities:				
Common stock issuable under the Company's equity incentive plans	—	—	2,043	889
Common stock issuable under the Company’s convertible debt	—	—	—	4,365
Weighted-average common shares outstanding, diluted	193,590	192,032	195,214	196,893
Earnings (loss) per common share, basic	$ (0.63)	$ (0.16)	$ 0.14	$ 2.06
Earnings (loss) per common share, diluted	$ (0.63)	$ (0.16)	$ 0.14	$ 2.04

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,	
	2026	2025	2026	2025
Common stock issuable under the Company's equity incentive plans	14,407	12,390	12,364	11,501
Common stock issuable under the Company’s convertible debt	4,365	4,365	4,365	—
Total number of potentially issuable shares	18,772	16,755	16,729	11,501

(14) 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.

License Agreements

As part of the Amicus acquisition, the Company assumed the existing license agreement with Dimerix Limited (Dimerix) for the U.S. commercialization rights for BMN 820 (also known as DMX-200) for the treatment of FSGS and other indications. Pursuant to the agreement, if future development, regulatory or commercial milestones are met, the Company will be obligated to pay Dimerix up to a maximum aggregate amount of $510.0 million with respect to FSGS and tiered royalties of BMN 820 net sales in the U.S. ranging from the low-teens to low-twenties. Refer to Note 2 - Acquisitions for additional information on the acquisition.

As of September 30, 2026, the Company was also subject to contingent payments, primarily comprised of development, regulatory and commercial milestones relating to certain other license agreements. Those considered reasonably possible totaled $485.5 million and, of this amount, the Company may pay up to $7.5 million over the next twelve months if certain contingencies are met.

Other Commitments

The Company uses experts and laboratories at universities and other institutions to perform certain R&D activities. These amounts are recorded as R&D expense as services are provided. In the normal course of business, the Company enters into various firm purchase commitments primarily to procure active pharmaceutical ingredients, certain inventory-related items and certain third-party R&D services, production services and facility construction services. As of September 30, 2026, such commitments were estimated at $768.8 million, of which $291.1 million is expected to be paid in 2026, with the remainder to be paid in 2027, 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.

(15) SUBSEQUENT EVENTS

In October 2026, the Company entered into an exclusive worldwide license agreement with GC Biopharma Corp. (GC Biopharma) and Hanmi Pharm Co., Ltd. (Hanmi) to develop and commercialize BMN 450 (also known as HM15421/GC1134A), an investigational long-acting enzyme replacement therapy for Fabry disease. Under the terms of the agreement, the Company will make an upfront payment of $70.0 million, which it expects to record as acquired IPR&D expense in the fourth quarter of 2026. GC Biopharma and Hanmi are also jointly eligible to receive up to an additional $405.0 million upon the completion of certain development and commercial milestones, and tiered royalties.

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. Founded in 1997, our San Rafael, California-based company has a proven track record of innovation, with nine 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.

During 2026, we completed the acquisitions of Amicus Therapeutics, Inc. (Amicus) and Alesta Therapeutics B.V. (Alesta). The accounting impacts of these transactions have been reflected in the Condensed Consolidated Financial Statements. Refer to Note 2 – *Acquisitions* for additional details related to these acquisitions.

A summary of our commercial products, as of September 30, 2026, is provided below:

Commercial Products[1]	**Indication**
VOXZOGO (vosoritide)	Achondroplasia
Metabolic Conditions (formerly Enzyme Therapies):	
ALDURAZYME (laronidase)	MPS I
BRINEURA (cerliponase alfa)	Neuronal ceroid lipofuscinosis type 2 (CLN2)
GALAFOLD (migalastat HCl)[2]	Fabry
NAGLAZYME (galsulfase)	MPS VI
PALYNZIQ (pegvaliase-pqpz)	Phenylketonuria (PKU)
POMBILITI + OPFOLDA (cipaglucosidase alfa-atga/miglustat)[2]	Pompe
VIMIZIM (elosulfase alpha)	Mucopolysaccharidosis (MPS) IVA
KUVAN (sapropterin dihydrochloride)	PKU

(1) In 2026, we announced that we will no longer market ROCTAVIAN. For additional information related to ROCTAVIAN, see Note 19 - *Restructuring* to the Consolidated Financial Statements accompanying our Annual Report on Form 10-K for the year ended December 31, 2025.

(2) We acquired two commercial products, GALAFOLD and POMBILITI + OPFOLDA, in connection with the acquisition of Amicus on April 27, 2026. Refer to Note 2 - *Acquisitions* to our accompanying Condensed Consolidated Financial Statements for additional information.

Financial Highlights

Key components of our results of operations include the following:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Total revenues	$ 1,024.6	$ 776.1	$ 2,780.6	$ 2,346.7
Cost of sales	$ 205.4	$ 140.1	$ 603.2	$ 441.7
Research and development (R&D)	$ 180.5	$ 188.5	$ 566.3	$ 508.6
Acquired in-process research and development (IPR&D)	$ 287.7	$ 221.0	$ 287.7	$ 221.0
Selling, general and administrative (SG&A)	$ 333.6	$ 268.4	$ 987.5	$ 706.8
Intangible asset amortization	$ 99.5	$ 4.8	$ 177.4	$ 14.5
Interest expense	$ 55.6	$ 2.6	$ 133.9	$ 8.1
Provision for (benefit from) income taxes	$ (8.9)	$ 4.4	$ 43.4	$ 114.2
Net income (loss)	$ (122.8)	$ (30.7)	$ 27.5	$ 395.5

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, wars and military conflicts, 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 2026. 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 November 2026, we announced that the U.S. Food and Drug Administration (FDA) set a Prescription Drug User Fee Act (PDUFA) target action date of May 30, 2027 for supplemental new drug application (sNDA) to expand the use of VOXZOGO for the treatment of hypochondroplasia (HCH). We also announced that the European Medicines Agency (EMA) validated our submission for the approval of VOXZOGO to treat HCH.

- In October 2026, we entered into an exclusive worldwide license agreement with GC Biopharma Corp. (GC Biopharma) and Hanmi Pharm Co., Ltd. (Hanmi) to develop and commercialize BMN 450 (also known as HM15421/GC1134A), an investigational long-acting enzyme replacement therapy for Fabry disease. Under the terms of the agreement, we will make an upfront payment of $70.0 million that will be recorded as acquired IPR&D expense in the fourth quarter of 2026. GC Biopharma and Hanmi are also jointly eligible to receive up to an additional $405.0 million upon the completion of certain development and commercial milestones, and tiered royalties.

- In August 2026, we completed the acquisition of Alesta, a Dutch private limited liability company. The acquisition is intended to strengthen our Skeletal Conditions pipeline by adding BMN 440 (formerly ALE1), an orally active, small molecule for the potential treatment of hypophosphatasia (HPP). We accounted for this transaction as an asset acquisition since the lead asset, BMN 440, represents substantially all of the fair value of the gross assets acquired. See Note 2 to our accompanying Condensed Consolidated Financial Statements for additional information related to Alesta acquisition.

- In August 2026, we made the decision to discontinue development of BMN 401 across all indications.

- In July 2026, we announced that the FDA accepted our sNDA for full approval of VOXZOGO in children with achondroplasia, with a PDUFA target action date of February 28, 2027.

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,		
	2026	2025	Change	2026	2025	Change
VOXZOGO	$ 245.0	$ 218.4	$ 26.6	$ 717.4	$ 653.5	$ 63.9
Metabolic Conditions:						
VIMIZIM	215.8	182.8	33.0	620.4	586.5	33.9
NAGLAZYME	107.8	122.0	(14.2)	373.2	365.2	8.0
PALYNZIQ	111.0	108.8	2.2	335.8	308.0	27.8
GALAFOLD	151.8	—	151.8	257.6	—	257.6
BRINEURA	52.7	48.0	4.7	151.2	137.0	14.2
ALDURAZYME	56.6	53.7	2.9	136.9	159.1	(22.2)
POMBILITI + OPFOLDA	48.6	—	48.6	78.8	—	78.8
KUVAN	23.1	23.9	(0.8)	71.2	76.2	(5.0)
ROCTAVIAN	0.7	3.2	(2.5)	15.1	22.9	(7.8)
Total net product revenues	$ 1,013.1	$ 760.8	$ 252.3	$ 2,757.6	$ 2,308.4	$ 449.2

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, 2026 as compared to the three months ended September 30, 2025 was primarily attributed to the following:

- GALAFOLD and POMBILITI + OPFOLDA: revenues from commercial products acquired on April 27, 2026;
- VIMIZIM: higher sales volume due to timing of large international orders; and
- VOXZOGO: higher sales volume from new patients initiating therapy, primarily outside of the U.S.

These increases were partially offset by the following:

- NAGLAZYME: lower sales volume due to timing of large international orders.

The increase in Net Product Revenues for the nine months ended September 30, 2026 as compared to the nine months ended September 30, 2025 was primarily attributed to the following:

- GALAFOLD and POMBILITI + OPFOLDA: revenues from commercial products acquired on April 27, 2026;
- VOXZOGO: higher sales volume from new patients initiating therapy, primarily outside of the U.S.;
- VIMIZIM: higher sales volume due to timing of large international orders; and

- PALYNZIQ: higher sales volume from new patients initiating therapy, primarily in the U.S.

These increases were partially offset by the following:

- ALDURAZYME: lower sales volume due to timing of order fulfillment to Sanofi.

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 VOXZOGO, GALAFOLD and POMBILITI + OPFOLDA, see also the risk factors "Our success depends on our ability to manage our growth and execute our corporate strategy." and "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" 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, and 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 (USD), 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,		
	2026	2025	Change	2026	2025	Change
Sales denominated in USD	$ 454.4	$ 398.6	$ 55.8	$ 1,305.4	$ 1,160.6	$ 144.8
Sales denominated in foreign currencies	558.7	362.2	196.5	1,452.2	1,147.8	304.4
Total net product revenues	$ 1,013.1	$ 760.8	$ 252.3	$ 2,757.6	$ 2,308.4	$ 449.2

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Favorable (unfavorable) impact of foreign currency exchange rates on product sales denominated in currencies other than USD	$ 6.6	$ (8.2)	$ 14.8	$ 21.9	$ (28.9)	$ 50.8

The favorable impact for the three and nine months ended September 30, 2026 was primarily driven by strengthening of the Euro, partially offset by weakening of the Argentine Peso and Japanese Yen. 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.

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, charges for inventory write downs and amortization of acquired inventory fair value step-up recorded from business combinations.

The following table summarizes our Cost of Sales and Gross Margin:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Total revenues	$ 1,024.6	$ 776.1	$ 248.5	$ 2,780.6	$ 2,346.7	$ 433.9
Cost of sales	$ 205.4	$ 140.1	$ 65.3	$ 603.2	$ 441.7	$ 161.5
Gross margin	80.0 %	81.9 %	(1.9)%	78.3 %	81.2 %	(2.9)%

Cost of sales increased in the three and nine months ended September 30, 2026 as compared to the three and nine months ended September 30, 2025, primarily due to an increase in sales volume and amortization of acquired inventory fair value step-up. Gross Margin decreased in the three and nine months ended September 30, 2026 as compared to the three and nine months ended September 30, 2025, primarily due to unfavorable product mix driven by increased sales of lower-margin products.

Research and Development

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

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.

We continuously evaluate the recoverability of costs associated with pre-launch or pre-qualification manufacturing activities, if any, and capitalize the costs incurred related to those activities if we determine that recoverability is probable and therefore future revenues are expected. If the related product candidate's marketing application is rejected by the applicable regulators and the likelihood of future revenues for a product candidate become uncertain, the related manufacturing costs are expensed as R&D expenses.

R&D expense consisted of the following:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Research and early pipeline	$ 87.0	$ 93.7	$ (6.7)	$ 259.4	$ 280.2	$ (20.8)
Later-stage clinical programs	23.7	30.7	(7.0)	110.1	58.1	52.0
Marketed products	69.8	64.1	5.7	196.8	170.3	26.5
Total R&D expense	$ 180.5	$ 188.5	$ (8.0)	$ 566.3	$ 508.6	$ 57.7

The decrease in R&D expense for the three months ended September 30, 2026 as compared to the three months ended September 30, 2025 was primarily due to programs discontinued during the year, partially offset by spend related to the marketed products acquired from Amicus. The increase in R&D expense for the nine months ended September 30, 2026 as compared to the nine months ended September 30, 2025 was primarily due to higher spend on BMN 401, a later-stage clinical program that was discontinued in the third quarter of 2026, and spend related to the marketed products acquired from Amicus, partially offset by lower spend on Research and early pipeline due to program discontinuations.

Acquired IPR&D

Acquired IPR&D expenses are recorded when incurred and reflect costs of externally-developed IPR&D projects, acquired directly in a transaction other than a business combination, that do not have an alternative future use, including upfront and pre-commercialization milestone payments related to various license agreements and the costs of rights to IPR&D projects.

Acquired IPR&D was as follows:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Acquired IPR&D	$ 287.7	$ 221.0	$ 66.7	$ 287.7	$ 221.0	$ 66.7

The increase in Acquired IPR&D for the three and nine months ended September 30, 2026 as compared to the three and nine months ended September 30, 2025 was due to higher costs paid for BMN 440 in the third quarter of 2026 as compared to costs paid for BMN 401 in the third quarter of 2025. We expect acquired IPR&D to increase in the fourth quarter of 2026 related to the upfront payment for BMN 450. See Note 15 - *Subsequent Events* to our accompanying Condensed Consolidated Financial Statements for additional information.

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,		
	2026	2025	Change	2026	2025	Change
S&M	$ 148.9	$ 131.3	$ 17.6	$ 416.0	$ 356.0	$ 60.0
G&A	184.7	137.1	47.6	571.5	350.8	220.7
Total SG&A expense	$ 333.6	$ 268.4	$ 65.2	$ 987.5	$ 706.8	$ 280.7

S&M expense consisted of the following:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Metabolic Conditions	$ 86.6	$ 60.9	$ 25.7	$ 225.7	$ 165.5	$ 60.2
VOXZOGO	43.0	46.1	(3.1)	136.1	123.5	12.6
Other	19.3	24.3	(5.0)	54.2	67.0	(12.8)
Total S&M expense	$ 148.9	$ 131.3	$ 17.6	$ 416.0	$ 356.0	$ 60.0

The increase in S&M expense for the three months ended September 30, 2026 as compared to the three months ended September 30, 2025 was primarily due to spend related to GALAFOLD and POMBILITI + OPFOLDA, which were acquired from Amicus on April 27, 2026, partially offset by lower spend on ROCTAVIAN as a result of our strategic decision in the fourth quarter of 2025 to voluntarily withdraw it from the market. The increase in S&M expense for the nine months ended September 30, 2026 as compared to the nine months ended September 30, 2025 was primarily due to spend related to GALAFOLD, POMBILITI + OPFOLDA, and VOXZOGO, partially offset by lower spend on ROCTAVIAN as mentioned above.

The increase in G&A expense for the three and nine months ended September 30, 2026 as compared to the three and nine months ended September 30, 2025 was primarily due to severance and integration charges related to the acquisition of Amicus and ongoing corporate initiatives.

Intangible Asset Amortization

Intangible Asset Amortization was as follows:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Amortization of intangible assets	$ 99.5	$ 4.8	$ 94.7	$ 177.4	$ 14.5	$ 162.9

The increase in Amortization of intangible assets for the three and nine months ended September 30, 2026 as compared to the three and nine months ended September 30, 2025 was primarily due to the amortization of the intangible assets acquired from Amicus on April 27, 2026. With the addition of the acquired intangible assets, we expect amortization of intangible assets to increase over the next 12 months.

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,		
	2026	2025	Change	2026	2025	Change
Interest income	$ 4.6	$ 17.9	$ (13.3)	$ 37.7	$ 55.7	$ (18.0)

The decrease in Interest income for the three and nine months ended September 30, 2026 as compared to the three and nine months ended September 30, 2025 was primarily due to lower cash and investment balances as investments were liquidated to fund a portion of the Amicus transaction. We expect Interest income to decrease over the next 12 months due to lower cash and investment balances.

Interest Expense

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Interest expense	$ 55.6	$ 2.6	$ 53.0	$ 133.9	$ 8.1	$ 125.8

The increase in Interest expense for the three and nine months ended September 30, 2026 as compared to the three and nine months ended September 30, 2025 was primarily due to borrowings obtained to finance a portion of the Amicus transaction in April 2026. The increase in Interest expense for the nine months ended September 30, 2026 was also partially attributable to bridge commitment fees that were amortized to Interest expense following the termination of the bridge commitment in the first half of 2026. We expect Interest expense to increase over the next 12 months due to long-term debt financings completed in 2026. See Note 7 - *Debt* 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,		
	2026	2025	Change	2026	2025	Change
Other income, net	$ 1.3	$ 5.1	$ (3.8)	$ 8.6	$ 8.0	$ 0.6

The decrease in Other Income, Net for the three months ended September 30, 2026 as compared to the three months ended September 30, 2025 was primarily due to the absence of insurance proceeds related to damaged goods received in the third quarter of 2025 with no comparable proceeds in 2026. Other Income, Net for the nine months ended September 30, 2026 as compared to the nine months ended September 30, 2025 was relatively flat.

Provision for (Benefit from) Income Taxes

The Provision for (Benefit from) Income Taxes for the periods presented was as follows:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Provision for (benefit from) income taxes	$ (8.9)	$ 4.4	$ (13.3)	$ 43.4	$ 114.2	$ (70.8)

The decrease in Provision for (Benefit from) Income Taxes for the three and nine months ended September 30, 2026 as compared to the three and nine months ended September 30, 2025 was primarily due to lower pre-tax income driven by non-deductible acquired IPR&D charges and expenses attributed to the Amicus acquisition, which include increased interest expense and amortization of the acquired intangible assets. The impact of non-deductible acquired IPR&D on the annual effective tax rate was greater in 2026 as compared to 2025, resulting in a benefit from income taxes for the three months ended September 30, 2026.

Financial Condition, Liquidity and Capital Resources

Our cash, cash equivalents and investments as of September 30, 2026 and December 31, 2025 were as follows:

	September 30, 2026	December 31, 2025	Change
Cash and cash equivalents	$ 736.0	$ 1,311.7	$ (575.7)
Short-term investments	—	248.9	(248.9)
Long-term investments	—	492.2	(492.2)
Total cash, cash equivalents and investments	$ 736.0	$ 2,052.8	$ (1,316.8)

We believe cash generated from sales of our commercial products, in addition to our cash, cash equivalents and external financings, will be sufficient to satisfy our liquidity requirements for at least the next 12 months, including our debt service commitments relating to the Amicus acquisition. 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. 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 outstanding indebtedness, 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 long-term debt in cash. In addition, depending on prevailing market conditions, our liquidity requirements, contractual restrictions, and other factors, we may also from time to time seek to retire or purchase our outstanding convertible debt through cash purchases and/or exchanges for equity securities, in open market purchases, privately negotiated transactions or otherwise.

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, wars and military conflicts, 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,		
	2026	2025	Change
Net cash provided by operating activities	$ 577.0	$ 728.4	$ (151.4)
Net cash used in investing activities	$ (4,683.2)	$ (371.8)	$ (4,311.4)
Net cash provided by (used in) financing activities	$ 3,532.7	$ (45.6)	$ 3,578.3

The decrease in net cash provided by operating activities in the nine months ended September 30, 2026 compared to September 30, 2025 was primarily attributed to a decrease in Net Income and timing of cash payments to vendors, partially offset by a decrease in inventory purchases and timing of cash receipts from our customers.

The increase in net cash used in investing activities in the nine months ended September 30, 2026 compared to September 30, 2025 was primarily attributable to net cash paid for acquisitions of Amicus and Alesta, partially offset by higher net maturities of marketable securities.

The increase in net cash provided by financing activities in the nine months ended September 30, 2026 compared to September 30, 2025 was primarily attributable to proceeds from the secured term loan facilities and the 2034 Notes, offset by partial repayment of term loan facilities.

Financing

As of September 30, 2026, we had approximately $4.2 billion (undiscounted) aggregate principal amount of indebtedness, which requires and will require material cash payments for interest and principal. Our indebtedness consisted of:

- $600.0 million aggregate principal amount of 1.25% senior subordinated convertible notes due May 2027 (the 2027 Notes);
- $850.0 million aggregate principal amount of 5.5% senior unsecured notes due February 2034;
- $790.0 million outstanding under a senior secured term loan A facility maturing in April 2031 (the Term Loan A Facility); and
- $1,995.0 million outstanding under a senior secured term loan B facility maturing in April 2033 (the Term Loan B Facility, and, together with the Term Loan A Facility, the Term Facilities).

2034 Notes and Term Facilities

In April 2026, we used the net proceeds from the 2034 Notes, borrowings under the Term Facilities and approximately $1.7 billion of cash on hand to fund the consideration payable in connection with the Amicus acquisition, totaling approximately $5.3 billion. As a result of the acquisition financing, the aggregate principal amount of our indebtedness increased from approximately $1.5 billion as of March 31, 2026 to approximately $4.2 billion as of September 30, 2026.

Our increased indebtedness has increased our cash requirements, including fixed-rate interest payments on the 2027 Notes and 2034 Notes, variable-rate interest payments and scheduled principal payments under the Term Facilities. The 2027 Notes mature in May 2027 and, to the extent not converted, repurchased, refinanced or otherwise settled before maturity, will be required to be paid in cash. The Term Loan A Facility requires quarterly principal repayments equal to 1.25% of the original principal amount, with the remaining outstanding principal balance due at maturity. The Term Loan B Facility requires quarterly principal repayments equal to 0.25% of the original principal amount, with the remaining outstanding principal balance due at maturity. See also "In addition, our ability to refinance our indebtedness will depend on capital markets and our financial condition at such time" in "Risk Factors" included in Part II, Item 1A of this Quarterly Report.

Revolving Facility

In April 2026, we also entered into a $600.0 million senior secured revolving credit facility maturing in April 2031 (the 2026 Revolving Facility and, together with the Term Facilities, the 2026 Senior Secured Credit Facilities). As of September 30, 2026, $600.0 million was available under the 2026 Revolving Facility subject to satisfaction of the applicable borrowing conditions under the credit agreement governing the 2026 Senior Secured Credit Facilities (the 2026 Credit Agreement), for working capital and general corporate purposes. As of September 30, 2026, there were no amounts outstanding under the 2026 Revolving Facility.

Interest Rates on the 2026 Senior Secured Credit Facilities

Borrowings under the Term Facilities bear interest, at our option, at either (a) an alternate base rate, which is defined as a fluctuating rate per annum equal to the greatest of (i) the prime rate then in effect, (ii) the federal funds effective rate then in effect plus 0.50% per annum, (iii) a term Secured Overnight Financing Rate (SOFR) rate determined on the basis of a one-month interest period plus 1.00% per annum and (iv) 1.00%, in each case, plus the applicable margin, or (b) a term SOFR rate based on a one-, three- or six-month interest period, plus the applicable margin. The applicable margin for borrowings under the Term Loan B Facility is 0.75% for alternate base rate borrowings and 1.75% for term SOFR borrowings. The applicable margin for borrowings under the Term Loan A Facility is initially 0.75% for alternate base rate borrowings and 1.75% for term SOFR borrowings and, beginning on the first business day immediately following delivery of the compliance certificate for the first full fiscal quarter ending after April 27, 2026, ranges from 0.00% to 0.75% for alternate base rate borrowings and from 1.00% to 1.75% for term SOFR borrowings, in each case based on our total net leverage ratio. Dollar-denominated borrowings under the 2026 Revolving Facility, if any, bear interest on the same basis and at the same applicable margins as borrowings under the Term Loan A Facility. Interest on alternate base rate borrowings is payable quarterly in arrears on the last business day of March, June, September and December and on the applicable maturity date. Interest on term SOFR borrowings is payable in arrears on the last day of the applicable interest period or, if earlier, on the maturity date of the applicable facility and, for any interest period exceeding three months, every three months after the beginning of such interest period. As a result, our interest expense and cash requirements under the Term Facilities will vary based on changes in applicable benchmark interest rates. See also "Interest Rate Market Risk" in "Quantitative and Qualitative Disclosures About Market Risk" included in Part I, Item 3 of this Quarterly Report.

Credit Agreement Covenants

Our obligations under the 2026 Credit Agreement are guaranteed by certain of our subsidiaries and secured by first-priority liens on substantially all of our and the guarantors' assets, subject to specified exceptions. The 2026 Credit Agreement contains customary affirmative and negative covenants, including covenants that restrict, subject to specified exceptions, our ability to incur additional indebtedness, create liens, make investments, pay dividends or make other restricted payments, dispose of assets and enter into transactions with affiliates. In addition, the Term Loan A Facility and 2026 Revolving Facility require us to maintain a maximum Total Net Leverage Ratio (as defined in the 2026 Credit Agreement) of 3.50 to 1.00, subject to a temporary increase to 4.00 to 1.00 in connection with certain acquisitions, and a minimum Interest Coverage Ratio (as defined in the 2026 Credit Agreement) of 3.00 to 1.00. As of September 30, 2026, we are in compliance with these covenants.

See Note 7 - *Debt* to our Condensed Consolidated Financial Statements for additional information regarding our indebtedness and the 2026 Senior Secured Credit Facilities and Note 10 to the Consolidated Financial Statements accompanying our Annual Report on Form 10-K for the year ended December 31, 2025.

Material Cash Requirements

Material Indebtedness

Our increased indebtedness has increased our cash requirements, including fixed-rate interest payments on the 2027 Notes and 2034 Notes, variable-rate interest payments and scheduled principal payments under the Term Facilities. See Note 7 - *Debt* to our Condensed Consolidated Financial Statements for additional information regarding our indebtedness.

Purchase and Lease Obligations, and Unrecognized Tax Benefits

As of September 30, 2026, we had obligations of approximately $768.8 million, of which $291.1 million is expected to be paid in 2026 and the remainder in 2027. 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.

Our lease commitments and unrecognized tax benefits as of September 30, 2026 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, 2025.

See Note 14 - *Commitments and Contingencies* 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, 2026, 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, 2025, filed with the SEC on February 26, 2026.

Recent Accounting Pronouncements

See Note 1 - *Business Overview and Significant Accounting Policies* 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, 2026 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, 2025, except as described below.

Interest Rate Market Risk

Our exposure to market risk for changes in interest rates relates primarily to our cash, cash equivalents, investments, and our variable-rate term loans. The interest rate risk related to our variable-rate term loans arises from these bearing interest based on Secured Overnight Financing Rate (SOFR) or an alternate base rate plus a margin. As of September 30, 2026, our outstanding debt included $2.8 billion of variable-rate debt on Term Facilities. Assuming a 100 basis point increase in the applicable interest rates, annual interest expense on existing variable-rate debt would be expected to increase by approximately $27.9 million. The remaining outstanding debt as of September 30, 2026, was fixed-rate debt and is not exposed to risk related to changes in interest rates.

There were no borrowings under the 2026 Revolving Facility as of September 30, 2026.

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, 2026.

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 below, 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 global enterprise resource planning (ERP) system at certain subsidiaries, replacing existing operating and financial systems. The final phase of the deployment was completed in January 2026. 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 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.

On April 27, 2026, we completed our acquisition of Amicus Therapeutics, Inc. (Amicus), which was accounted for as a business combination using the acquisition method of accounting. The results of operations of the acquired Amicus business have been included in our results of operations since April 27, 2026. We are currently in the process of evaluating and integrating Amicus' historical internal controls into our control environment.

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, 2025, which was filed with the SEC on February 26, 2026.

Business and Operational Risks

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

Our corporate strategy is focused on innovation, growth, and value commitment, which includes, among other things, the acceleration or discontinuation of certain programs, the continued 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 are continuing to advance VOXZOGO for the treatment of conditions beyond achondroplasia, including hypochondroplasia and idiopathic short stature. 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. We are also expanding our pipeline through external innovation. For example, in April 2026, we acquired Amicus Therapeutics, Inc. (Amicus) to expand and diversify our rare disease product portfolio (the Amicus Acquisition) and in August 2026, we acquired Alesta Therapeutics B.V. (Alesta) to strengthen our skeletal conditions portfolio. In order to continue the development of our 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 have in the past attempted and may in the future 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 corporate strategy, we have acquired, entered into agreements to acquire, and may continue to acquire, companies or businesses that we believe could complement, expand or enhance our product offerings. For example, in April 2026, we acquired Amicus to expand and diversify our rare disease product portfolio and in August 2026, we acquired Alesta to strengthen our skeletal conditions portfolio. 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 have in the past and may in the future face risks or experience difficulties successfully integrating acquired businesses, such as Amicus or Alesta, 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. See "—Risks Related to the Amicus Acquisition" in this section for additional risks related to the Amicus Acquisition.

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. For example, we have incurred significant indebtedness in connection with the Amicus Acquisition. In addition, as experienced with BMN 401, acquired product candidates have not and may not perform as expected, may not result in regulatory approval, 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, we face competition from other companies that have developed, and may in the future develop, products for the treatment of achondroplasia. In addition, we face competition with respect to GALAFOLD and POMBILITI + OPFOLDA from several large pharmaceutical and biotechnology companies that currently market and sell products for the treatment of Fabry disease and Pompe disease. 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. In addition, we have and may in the future face challenges outside of the U.S. from competitors seeking to develop generic versions of GALAFOLD. 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.

For example, we faced significant uncertainty as to whether gene therapy would gain the acceptance of the public or the medical community. In October 2025, we announced our plan to pursue options to divest ROCTAVIAN, including exploring out-licensing opportunities. Subsequently in December 2025, we committed to a plan to voluntarily withdraw ROCTAVIAN from the market due to lower than previously anticipated commercial opportunities. In connection with this strategic decision, we recorded approximately $240.0 million of restructuring charges in 2025 comprised of an inventory write-off, impairment of long-lived assets, severance and other cost.

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, 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, either directly or through an acquisition, such as Amicus' collaboration agreement with respect to GALAFOLD. 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.

Risks Related to the Amicus Acquisition

***We may not realize the anticipated benefits from the Amicus Acquisition or accurately forecast the future performance of the combined company.**

The success of the Amicus Acquisition will depend, in part, on our ability to realize the anticipated benefits from successfully combining our and Amicus' businesses. We have and plan to continue to devote substantial management attention and resources to integrating our and Amicus' businesses so that we can fully realize the anticipated benefits of the Amicus Acquisition. This integration process may be disruptive to our and Amicus' businesses, and, if implemented ineffectively, could restrict realization of the expected benefits of the Amicus Acquisition. In addition, the acquired Amicus business, including GALAFOLD and POMBILITI + OPFOLDA, may not be successful, may require greater resources and investments than originally anticipated or may result in the assumption of unknown or contingent liabilities, which could have an adverse effect on us or our results of operations and our financial guidance.

Potential difficulties we may encounter include the following:

- the inability to successfully combine our and Amicus' businesses in a manner that permits us to realize the anticipated benefits of the Amicus Acquisition in the timeframe currently anticipated or at all;
- the failure to integrate internal systems, programs and internal controls, or applying different accounting policies, assumptions or judgments to Amicus' operational results than Amicus applied in the past;
- the inability to successfully obtain regulatory approval in new markets for, and continue to commercialize, GALAFOLD or POMBILITI + OPFOLDA on the currently anticipated timeline or at all;
- the inability to successfully commercialize and continue the commercial growth of GALAFOLD and POMBILITI + OPFOLDA;
- the inability to effectively and efficiently integrate information technology and other systems;
- issues not discovered as part of the transactional due diligence process or unanticipated liabilities or contingencies of Amicus, including employment or severance-related obligations under applicable law or other benefits arrangements, claims by or amounts owed to vendors or other commercial disputes, cyber incidents and information technology failures or delays, matters related to data privacy, data localization and the handling of personally identifiable information, intellectual property-related claims, including Hatch-Waxman litigation, and other unknown or contingent liabilities;
- preserving the important licensing, marketing, supply, distribution, and other commercial relationships of Amicus;
- the complexities associated with managing the combined company;
- the failure to retain key employees of either of the two companies who may be difficult to replace;
- the disruption of each company's ongoing businesses or inconsistencies in services, standards, controls, procedures and policies;
- potential unknown liabilities and unforeseen increased expenses, delays or regulatory conditions associated with the Amicus Acquisition; and
- performance shortfalls at one or both of the two companies as a result of the diversion of management's attention caused by completing the Amicus Acquisition and integrating our and Amicus' operations.

Any of these risks could adversely affect our ability to maintain relationships with collaboration partners, vendors, employees and other commercial relationships or adversely affect our future operational results. As a result, the anticipated benefits of the Amicus Acquisition may not be realized fully or at all or may take longer to realize or cost more than expected, which could adversely affect our business, financial condition, including our ability to generate sufficient cash to service our indebtedness, including our 1.25% senior subordinated convertible notes due in 2027 (the 2027 Notes), our 5.5% senior unsecured notes due in 2034 (the 2034 Notes and together with the 2027 Notes, the Notes) and any loans outstanding under the 2026 Credit Agreement (as defined below), results of operations, growth prospects and our financial guidance, and we may be forced to take other actions to satisfy our obligations under our indebtedness, which may not be successful. In addition, changes in laws and regulations could adversely impact our business, financial condition, results of operations and growth prospects. Furthermore, the risks discussed above could also adversely affect our ability to accurately forecast the operational and financial performance of the combined

company, including key financial metrics and prospects. If we are unable to accurately forecast and meet our financial guidance, our stock price can be materially adversely affected.

***We have incurred and expect to incur material expenses related to the Amicus Acquisition.**

We have incurred and expect to incur material expenses in connection with the Amicus Acquisition and the subsequent integration of the business, operations, practices, policies and procedures of Amicus. These additional expenses could have an adverse effect on us or our results of operations. While we have assumed that a certain level of transaction and integration expenses would be incurred, there are a number of factors beyond our control that could affect the total amount or the timing of integration expenses. Many of the expenses that will be incurred, by their nature, are difficult to estimate accurately at the present time.

***We may not realize the anticipated cost savings from the Amicus Acquisition.**

The benefits that we expect to achieve as a result of the Amicus Acquisition will depend, in part, on our ability to realize anticipated cost savings, including resulting from expected operational synergies and global infrastructure efficiencies. Following the closing of the Amicus Acquisition, we believe that we will be able to, among other matters, save on our costs by being able to streamline administrative processes and harmonize global distribution of the two companies.

Our success in realizing these cost savings, and the timing of this realization, depends on many factors. Although we have consummated the Amicus Acquisition, this may not result in the full realization of the cost savings that we expect, either within the expected timeframe, or at all. In addition, we cannot assure you that the costs to achieve these cost savings will not be higher than we anticipated. Therefore, we cannot assure you that any anticipated cost savings will be achieved or that our estimates and assumptions will prove to be accurate. If our cost savings are less than our estimates or our cost savings initiatives adversely affect our business or cost more or take longer to implement than we project, or if our assumptions prove to be inaccurate, our results could be lower than we anticipate.

***Our actual financial positions and results of operations may differ materially from the publicly filed unaudited pro forma condensed combined financial information.**

The unaudited pro forma condensed combined financial information contained in our amended Current Report on Form 8-K/A filed on July 13, 2026 and the unaudited pro forma financial information included in our Quarterly Reports on Form 10-Q for the quarter ended June 30, 2026 and September 30, 2026, are presented for illustrative purposes only and may differ materially from what our actual financial position or results of operations would have been had the Amicus Acquisition been completed on the dates indicated. The unaudited pro forma condensed combined financial information included in our amended Current Report on Form 8-K/A was derived from our and Amicus' audited and unaudited historical financial statements and certain adjustments and assumptions have been made regarding the combined company after giving effect to the Amicus Acquisition and related transactions. The assets and liabilities of Amicus were measured at fair value based on various preliminary estimates using assumptions that we believed were reasonable utilizing information currently available at the time such financial information was prepared. The process for estimating the fair value of acquired assets and assumed liabilities requires the use of judgment in determining the appropriate assumptions and estimates. These estimates may be revised as additional information becomes available and as additional analyses are performed. Differences between preliminary estimates reflected in the unaudited pro forma condensed combined financial information and preliminary acquisition accounting and the final acquisition accounting will occur and could have a material impact on the unaudited pro forma condensed combined financial information and the ultimate combined company's financial position and future results of operations.

In addition, the assumptions used in preparing the unaudited pro forma condensed combined financial information may not prove to be accurate, and other factors may affect our financial condition or results of operations following the closing of the Amicus Acquisition in April 2026.

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, the EU pharmaceutical reform will generally result in a decrease in data and market exclusivity 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 the relevant EU technical documentation assessment certificate, 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 marketed products have received regulatory approval to be commercially marketed and sold in the U.S., the EU, and certain other countries. Any product for which we have obtained regulatory approval, including the products acquired in the Amicus Acquisition, 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. Failure to continue to show favorable risk-benefit balance for BRINEURA could result in the withdrawal of the marketing approval.

Moreover, promotional communications with respect to 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, which is also subject to restrictions. 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, GALAFOLD and VOXZOGO are approved in the U.S. under accelerated approval and are subject to post-marketing requirements to obtain full approval. Continued approval 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 pricing, 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. For example, 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.

Several healthcare reform initiatives culminated in the enactment of the Inflation Reduction Act (IRA) in August 2022, which, among other things, eliminated, beginning in 2025, the coverage gap under Medicare Part D by significantly lowering the enrollee maximum out-of-pocket costs and requiring manufacturers to subsidize, through a newly established manufacturer discount program, 10% of Part D enrollees' prescription costs for brand drugs below the out-of-pocket limit, and 20% once the out-of-pocket limit has been reached. The IRA also requires the 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. The negotiated price may not exceed a statutory ceiling price. Only high-expenditure single-source drugs that have been approved for at least seven years (11 years for biologics) can be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product has been announced. In addition, CMS has selected and announced the negotiated maximum fair price for 15 additional Medicare Part D drugs which will become effective in 2027. For 2028, CMS has selected an additional 15 drugs, comprised of drugs covered under Medicare Part D and, for the first time, drugs payable under Medicare Part B. For 2029 and subsequent years, 20 Part B or Part D drugs will be selected. The IRA also imposes rebates on Medicare Part B and Part D drugs whose prices have increased at a rate greater than the rate of inflation, and in 2024, CMS finalized regulations for the Medicare Part B and Part D inflation rebates. 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.

In addition, the current U.S. Presidential Administration is pursuing policies to reduce regulations and expenditures across government including at HHS, which include the FDA and CMS, and related agencies. For example, on May 12, 2025, President Trump issued an executive order that, among other things, required HHS, within 30 days, to establish and communicate to drug manufacturers most favored nation (MFN) price targets designed to bring drug prices for American patients in line with those in comparably developed nations. If significant progress towards MFN pricing is not achieved, the executive order requires HHS to propose a rulemaking to implement MFN pricing. On December 23, 2025, CMS issued proposed regulations to establish, under the Center for Medicare and Medicaid Innovation, two mandatory MFN demonstration models under Medicare Parts B and D, respectively. If these rules or other MFN pricing rules are finalized, they are likely to reduce prices of at least some drugs in the United States, if they are also sold in comparator countries. Even if we do not market drugs in such countries, we will be indirectly affected if our drugs competed with drugs whose prices were reduced as a result of MFN pricing initiatives.

At the U.S. state level, legislatures 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 entered into force in January 2025 and has resulted 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. Under the current rules 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. Furthermore, it is expected that these periods will be shortened as a result of a reform of the EU pharmaceutical legislative package. On December 11, 2025, the European Parliament and the European Council reached a political agreement on the proposed revision of several European legislative instruments related to medicinal products, including orphan products. Among other things, the revision will amend the duration of the regulatory exclusivity. In particular, the regulatory data protection period (during which other companies cannot access product data) would amount to eight years, with one additional year of market protection (during which generic or biosimilar products cannot be sold), following an MA. Pharmaceutical companies would be eligible for additional periods of market protection under certain conditions, with a cap of eleven years on the combined regulatory protection period. Orphan medicinal products addressing a disease with no current available medicinal treatment (“breakthrough orphan medicinal products”) would benefit from up to eleven years of market exclusivity. These rules, if formally approved by the European Parliament and the European Council to become law, could adversely affect our products.

In addition, 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, 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, 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. 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. The FDA could also interpret the term “condition” narrowly, which could allow for indirect competition during the period of exclusivity. 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 clinically superior by means of greater safety or effectiveness by making a major contribution to patient care. Further, 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.**

ALDURAZYME, BRINEURA, NAGLAZYME, PALYNZIQ, VIMIZIM and POMBILITI 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 the EU, a biosimilar is typically defined as a biological medicine similar to another already approved biological medicine. Developers of biosimilars are required to demonstrate by the best possible means that their biological medicine is highly similar to the reference medicine in physicochemical and biological terms, notwithstanding natural variability inherent to all biological medicines; and that any observed differences are duly justified with regard to their potential impact on safety 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. The FDA has been changing the requirements for demonstrating biosimilarity, which may make it easier for biosimilars referencing our product to be licensed. 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. In the U.S., standards for interchangeability also are changing to make it easier for biosimilars to demonstrate interchangeability. 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 currently 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, including a reduction in some agencies' workforces and/or inadequate funding, 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 previously 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 recent shut down that began on October 1, 2025 and lasted until November 12, 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, 2026, we had cash and cash equivalents totaling $736.0 million and debt obligations of $4.2 billion (undiscounted), which consisted of $1.5 billion under the Notes, $1,995.0 million under a senior secured term loan B facility (the Term Loan B Facility) and $790.0 million under a senior secured term loan A facility (the Term Loan A Facility and, together with the Term Loan B Facility, the Term Facilities). In April 2026, we incurred indebtedness under the Term Facilities and entered into a $600.0 million senior secured revolving credit facility (the 2026 Revolving Facility and, together with the Term Facilities, the 2026 Senior Secured Credit Facilities). 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 Notes and under the credit agreement governing the 2026 Senior Secured Credit Facilities (the 2026 Credit Agreement), but also to repay the principal amounts associated with the 2027 Notes (if not converted), the 2034 Notes and any Loans (as defined therein) outstanding under the 2026 Credit Agreement.

We may require additional financing to fund the repayment of the Notes and the Loans, 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;
- our future plans for strategic investments and/or acquisitions;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the progress of research programs carried out by us;
- any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish;
- Sanofi's ability to continue to successfully commercialize ALDURAZYME; and
- whether our convertible debt is converted to common stock in the future.

Moreover, our fixed expenses such as rent, license payments, interest expense and other contractual commitments are substantial and may increase in the future. These fixed expenses may increase because we may enter into:

- additional licenses and collaborative agreements;
- additional contracts for product manufacturing; and
- additional financing facilities or arrangements.

We will need to raise additional funds from equity or debt securities, loans or collaborative agreements if we are unable to satisfy our liquidity requirements. The sale of additional equity and/or equity-linked securities will result in additional dilution to our stockholders. Furthermore, additional financing may not be available in amounts or on terms satisfactory to us or at all. This could result in the delay, reduction or termination of our research, which could harm our business.

***We have incurred in the past and may in the future incur 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, 2026, we had $4.2 billion (undiscounted) principal amount of indebtedness outstanding under the Notes and the Term Facilities. As of September 30, 2026, no amount was outstanding under the 2026 Revolving 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 indenture governing our 2034 Notes and the 2026 Credit Agreement contain, and any future indebtedness that we may incur may contain, financial and/or 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 2026 Credit Agreement or the indentures governing our Notes, the outstanding borrowings thereunder could become immediately due and payable, the 2026 Revolving Facility lenders could refuse to permit additional borrowings under the 2026 Revolving Facility, or it could lead to defaults under agreements governing our current or future indebtedness, including the 2026 Credit Agreement and the indentures governing the Notes, as applicable

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

As of September 30, 2026, our outstanding indebtedness consisted of the 2027 Notes, which, if not converted, will be required to be repaid in cash at maturity in May 2027, the 2034 Notes, which will be required to be repaid in cash at maturity in February 2034, and the Term Facilities. While we could seek to obtain additional third-party financing to pay for any amounts due in cash upon maturity of the Notes, we cannot be sure that such third-party financing will be available on commercially reasonable terms, if at all. If not sooner repaid, loans outstanding under our Term Loan B Facility will be required to be repaid in cash at maturity in April 2033 and loans outstanding under our Term Loan A Facility and 2026 Revolving Facility will be required to be repaid in cash at maturity in April 2031.

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, 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. We have in the past and may in the future face difficulty in expanding our manufacturing capabilities, which has and may contribute to increased costs and reduced margins.

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 candidates 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. Additionally, we have in the past experienced and may in the future experience strong demand that outpaces our projections and supply constraints. If our manufacturing capabilities are unable to meet demand, we may lose potential revenues with respect to our products or experience adverse impacts on our development programs.

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.

In addition, we currently rely on third parties for all or portions of the manufacture of our products. For example, GALAFOLD and POMBILITI are manufactured by single-source third-party manufacturers. 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;
- geopolitical instability resulting from war, terrorism and other violence; 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 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 tensions or conflicts, and economic instability;
- 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 of 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. Additionally, ongoing conflicts in the Middle East, including the conflict between the U.S. and Iran, have introduced additional geopolitical instability and uncertainty. We have operations in the Middle East, and this conflict has disrupted and may continue to disrupt our commercial operations and supply chain, which may have a material adverse impact on our ability to sell our products and/or collect receivables from customers in the region.

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 British Pound, the Canadian Dollar, the Argentine Peso, the Colombian Peso, the Mexican Peso and several other currencies, changes in those currencies relative to the U.S. Dollar (USD) have in the past and may in the future impact our revenues and expenses. If the USD were to weaken against another currency (as was 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, including with respect to Amicus, which has historically not engaged in any foreign exchange hedging activities, 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 individuals, entities, and their intermediaries from directly or indirectly making, offering, providing, promising, or authorizing provision of 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. Following that investigation, the President announced a proclamation which will impose 100% tariffs on certain patented

pharmaceutical products and associated pharmaceutical ingredients beginning September 29, 2026, unless modified by the President. On September 23, 2026, the U.S. Department of Commerce issued guidance to clarify the scope of these tariffs, including creating a process for seeking an exclusion for pharmaceutical products and associated ingredients, including orphan drugs, that meet an urgent U.S. health need. We are continuing to monitor the potential impact of the proclamation, the guidance and any additional enabling regulatory actions on our business. 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. Notwithstanding the U.S. Supreme Court's recent decision invalidating tariffs imposed under the International Emergency Economic Powers Act, 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, including as a result of litigation or other challenges, 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 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. 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.

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.

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 unpredictable outcomes, which can include the initiation of defensive proceedings against us in retaliation. We have and may in the future reach settlement agreements pursuant to which we license our intellectual property rights to third parties in exchange for royalties or other consideration. For example, in September 2026, we entered into a Settlement and License Agreement (the Ascendis Agreement) with Ascendis Pharma A/S (Ascendis), pursuant to which we settled all pending patent litigation with Ascendis and granted a license to our patents that relate to Ascendis’ product Yuviwel for all current and potential indications, including achondroplasia and hypochondroplasia, in exchange for royalties.

In addition, settlement agreements may limit our control over the prosecution, maintenance, enforcement or defense of intellectual property rights subject to such arrangements, and our licensees' interests may differ from ours. Disputes may also arise regarding the scope of the rights granted under these arrangements, the parties' respective obligations or the interpretation of other contractual provisions. Any such limitations or disputes could impair our ability to protect or enforce our intellectual property rights or otherwise adversely affect our business, financial condition and results of operations.

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 have an ownership interest in our patents, 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, have and may in the future 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.

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;

- strategic transactions, including acquisition of products, businesses, or other assets;
- generic competition from current and 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;
- 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 and the change of control repurchase feature of the 2034 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) and the terms of the 2034 Notes require us to offer to repurchase the 2034 Notes in the event of a change of control (as defined in the indenture governing the 2034 Notes). This may have the effect of delaying or preventing a takeover of us that would otherwise be beneficial to our stockholders or investors in the Notes.

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, as well as our ability to integrate employees who join us as the result of an acquisition or business combination. 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. Changes to 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. 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. We have in the past and may in the future announce reductions in our global workforce, which could lead to 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 in turn 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 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 subsequent 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 have made (and are expected to continue to make) important changes to the international tax system. These proposals include, among other measures, the imposition of 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 (with further provisions expected to be enacted in the future). The OECD has issued (and is expected to continue to issue further) administrative guidance providing transition and safe harbor rules in relation to the implementation of the Pillar Two proposal. For example, on January 5, 2026, the OECD published details of a proposed "side-by-side" arrangement providing for, among other things, additional safe harbors for multinational groups headquartered in certain qualifying jurisdictions, which includes the U.S. Based on the applicable thresholds, we are within the scope of the Pillar Two rules, but 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. We continue to monitor developments and are evaluating the potential impacts of the Pillar Two rules, including on our effective tax rates, and considering our eligibility to qualify for the relevant safe harbor rules (including under the proposed "side-by-side" arrangement).

Our ability to use net operating losses to offset future taxable income may be subject to certain limitations.

Under the provisions of the Internal Revenue Code of 1986, as amended (the Internal Revenue Code), changes in our ownership may limit the amount of pre-change net operating loss carryforwards (NOLs) that can be utilized annually in the future to offset taxable income. Section 382 of the Internal Revenue Code imposes limitations on a company's ability to use its NOLs to offset its taxable income if one or more stockholders or groups of stockholders that each own at least five percent of the company's stock increase their aggregate ownership (by value) by more than 50 percentage points over their lowest ownership percentages within a rolling three-year period. Similar rules may apply under state and foreign tax laws. Thus, changes in our ownership may limit our ability to use our NOLs. Subsequent statutory or regulatory changes in respect of the utilization of NOLs for federal, state or foreign purposes, such as suspensions on the use of NOLs or limitations on the deductibility of NOLs carried forward, or other unforeseen reasons, may result in our existing NOLs expiring or otherwise being unavailable to offset future income tax liabilities. For these reasons, we may not be able to utilize a material portion of our NOLs.

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), together with the California Privacy Rights Act (CPRA), requires covered businesses that process personal data of California residents to disclose their data collection, use and sharing practices. Further, the CCPA provides California residents with data privacy rights (including the ability to opt out of the sale of personal data), imposes new operational requirements for covered businesses, and provides for civil penalties for violations, as well as a private right of action for data

breaches that is expected to increase data breach litigation. The CPRA, among other things, gives California residents the ability to limit use of certain sensitive personal data, establishes restrictions on the retention of personal data, expands the types of data breaches subject to the CCPA's private right of action, and establishes a new California Privacy Protection Agency to implement and enforce the new law.

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 CCPA applies to personal data of consumers, business representatives, and employees who are California residents and 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 persons 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 across the world 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; interruptions or stoppages in our business operations (including clinical trials); 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, 2026, and aim to make it easier for certain victims to claim damages for certain 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 poses 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. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks.

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 have not and may not in the future, however, detect and remediate all such vulnerabilities including on a timely basis. Further, we have and may in the future 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 have in the past and may in the future cause a security incident or other interruption that have in the past and may in the future 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.

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.

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 VIMIZIM. This facility is 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, 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. 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 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, 2026, none of our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated any “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as those terms are defined in Item 408 of Regulation S-K.

Item 6. Exhibits

Exhibit Number	Description
2.1	Agreement and Plan of Merger, dated as of December 19, 2025, by and among BioMarin Pharmaceutical Inc., Amicus Therapeutics, Inc. and Lynx Merger Sub 1, Inc., previously filed with the SEC on December 19, 2025, as Exhibit 2.1 to the Company's Current Report on Form 8-K (File No.000-26727), which is incorporated herein by reference.
2.2†#	Share Purchase Agreement, dated as of August 17, 2026, by and among BioMarin Pharmaceutical Inc., Alesta Therapeutics B.V., each holder of shares of Alesta Therapeutics B.V. identified on Schedule 1.1(a) thereto, Anaheim SpinCo B.V. and Shareholder Representative Services LLC, solely in its capacity as the representative and attorney-in-fact of the Company Participating Equityholders, previously filed with the SEC on August 18, 2026, as Exhibit 2.1 to the Company's Current Report on Form 8-K (File No. 000-26727), 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 February 26, 2026 as Exhibit 3.2 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
10.1*†#	Settlement and License Agreement, made effective as of August 28, 2026, by and between BioMarin Pharmaceutical Inc. and Ascendis Pharma A/S.
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, 2026, are embedded within the Inline XBRL document.

* Filed herewith

† Certain exhibits and schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. the Company agrees to furnish supplementally a copy of any omitted exhibit or schedule to the SEC upon request; provided, however, that the Company may request confidential treatment pursuant to Rule 24b-2 of the Exchange Act for any schedule so furnished.

Portions of this exhibit have been omitted pursuant to Item 601(b)(2)(ii) and Item 601(b)(10)(iv) of Regulations S-K, as applicable. The Company agrees to furnish supplementally an unredacted copy of the exhibit to the SEC upon its request.

\+ 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, 2026 and December 31, 2025, (ii) Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2026 and 2025, (iii) Condensed Consolidated Statement of Stockholders' Equity for the three and nine months ended September 30, 2026 and 2025, (iv) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2026 and 2025, and (v) Notes to Condensed Consolidated Financial Statements (unaudited).

SIGNATURE

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

BIOMARIN PHARMACEUTICAL INC.

Dated: November 3, 2026

By /S/ BRIAN R. MUELLER

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

Exhibit 10.1

*****Certain identified information has been omitted from this exhibit because it is both (i) not material and (ii) of the type that the company treats as private or confidential. Such omitted information is indicated by brackets ("[***]") in this exhibit.*****

SETTLEMENT AND LICENSE AGREEMENT

This SETTLEMENT AND LICENSE AGREEMENT (this "**Agreement**") is made and effective as of August 28, 2026 (the "**Effective Date**"), by and between, on the one hand, BioMarin Pharmaceutical Inc. ("**BioMarin**"), and on the other hand, Ascendis Pharma A/S ("**Ascendis**"). BioMarin and Ascendis are together referred to as the "**Parties**," and each separately as a "**Party**."

RECITALS

WHEREAS, BioMarin is the owner of certain patents and patent applications relating to variants of C-type natriuretic peptide;

WHEREAS, Ascendis markets a pharmaceutical product containing the C-type natriuretic peptide known as "TransCon CNP," "navepegritide," or "YUVIWEL" with the specific sequences set forth in **Exhibit A**;

WHEREAS, BioMarin is the holder of a New Drug Application No. 214938 (the "**BioMarin NDA**"), which is approved by the U.S. Food and Drug Administration ("**FDA**") and pursuant to which it markets a competitive pharmaceutical product containing the C-type natriuretic peptide known as "VOXZOGO" or "vosoritide";

WHEREAS, the Parties are engaged in certain legal proceedings relating to the Licensed Product in various forums around the world, including those set forth on **Schedule 1** attached hereto;

WHEREAS, the Parties entered into that certain Binding Term Sheet for Settlement and License Agreement dated August 28, 2026 (the "**Binding Term Sheet**"), and in connection with the Binding Term Sheet, the Parties wish for the terms and conditions of this Agreement to replace and supersede the Binding Term Sheet in its entirety;

WHEREAS, the Parties wish to settle all Legal Proceedings on the terms and conditions set forth in this Agreement, and have commenced the process of such settlement as of the date of the Binding Term Sheet;

WHEREAS, the execution of this Agreement and the settlement of the Legal Proceedings contemplated herein do not constitute an admission by any Party as to liability, validity, infringement, enforceability, or damages; and

WHEREAS, this Agreement has been negotiated by the Parties at arm's length.

NOW, THEREFORE, in consideration of the mutual covenants, agreements, representations, and warranties set forth herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties agree as follows:

ARTICLE 1 DEFINITIONS

Section 1.1 **Certain Defined Terms**. The following terms, when used with initial capital letters, shall have the meanings set forth below:

"**Accounting Standards**" means, with respect to Ascendis, International Financial Reporting Standards (IFRS) or Generally Accepted Accounting Principles (GAAP) (and the maintenance of books and records prepared in accordance therewith), as applicable, depending on which accounting standards or principles (as applicable) are currently used by Ascendis or its Affiliates or sublicensees at the relevant time, in each case as consistently applied by Ascendis in its financial statements (if available) or as applied in their individual financial statements each as at the relevant balance sheet date.

"**Affiliate**" means any Person which, directly or indirectly, Controls another Person, is Controlled by another Person, or is under common Control with another Person.

"**Applicable Law**" means the applicable provisions of any and all national, supranational, regional, state, and local laws, treaties, statutes, rules, regulations, policies, administrative codes, guidance, ordinances, judgments, decrees, directives, injunctions, orders, permits of or from any governmental authority having jurisdiction over or related to the subject item, including the U.S. Food, Drug and Cosmetic Act (21 U.S.C. § 301 et seq.), Prescription Drug Marketing Act, the Generic Drug Enforcement Act of 1992 (21 U.S.C. § 335a et seq.), U.S. Patent Act (35 U.S.C. § 1 et seq.), Federal Civil False Claims Act (31 U.S.C. § 3729 et seq.), and the Anti-Kickback Statute (42 U.S.C. § 1320a-7b), all as amended from time to time, together with any rules, regulations, policies, and compliance guidance promulgated thereunder.

"**Ascendis Indemnitees**" has the meaning set forth in Section 7.2. "**BioMarin Indemnitees**"

has the meaning set forth in Section 7.1. "**BioMarin NDA**" has the meaning set forth in the

recitals.

"**BioMarin Other Patents**" means, other than the Licensed Patents, (i) any other patents in any jurisdiction worldwide, the claim(s) of which cover the Licensed Product, that are not patents at issue in any of the Legal Proceedings; and (ii) any patent that issues out of a current or future patent application, and any corrections, extensions, reissues, or reexaminations of such other patents or amended claims of such other patents arising out of an inter partes review, post-grant review, or other patent proceeding in any jurisdiction, in each case, clauses (i) and (ii) are limited to such patents that are controlled by BioMarin or any of its Affiliates now or in the future that claim or cover the making, using, storing, having stored, selling, offering for sale, or importation of the Licensed Product.

"**BioMarin Patent Rights**" means (i) the Licensed Patents and (ii) the BioMarin Other Patents.

"**Binding Term Sheet**" has the meaning set forth in the recitals.

"**Calendar Quarter**" means each three-month period ending on March 31, June 30, September 30, and December 31 of each Calendar Year.

"**Calendar Year**" means each successive period of twelve (12) calendar months commencing on January 1 and ending on December 31, except that the first Calendar Year of the Term shall commence on the Effective Date and end on December 31 of the year in which the Effective Date occurs and the last Calendar Year of the Term shall commence on January 1 of the year in which the Term ends and end on the last day of the Term.

"**Combination Product**" means any pharmaceutical or biological preparation that (a) contains or incorporates a Licensed Product in combination with one (1) or more other active pharmaceutical ingredients, and (b) is co-formulated or co-packaged or otherwise sold as a unit at a single price. For clarity, the Licensed Product in a Combination Product is licensed pursuant to this Agreement.

"**Confidential Information**" has the meaning set forth in Section 5.1.

"**Control**" means having the actual present capacity to elect a majority of the directors, or the power to direct greater than fifty percent (50%) of the voting rights entitled to elect directors of such Person; provided, however, that with respect to any Person in a country where the local law will not permit majority foreign equity participation, "Control" means with respect to the determination of an Affiliate, the ownership or control (directly or indirectly) of the maximum percentage of such outstanding stock or voting rights permitted by local law. A Person will be deemed an Affiliate of Ascendis solely for the term during which it satisfies the foregoing definition.

"**Dispute**" has the meaning set forth in Section 9.2. "**Effective Date**" has the

meaning set forth in the preamble. "**FDA**" has the meaning set forth in the

recitals.

"**First Commercial Sale**" means, (i) with respect to Ascendis' YUVIWEL product, the first commercial sale thereof in the United States (i.e., April 6, 2026), and (ii) with respect to any country other than the United States within the Territory, the date of first commercial sale of a Licensed Product (i.e., any sale or other disposition of such Licensed Product that results in revenue to Ascendis) therein, as applicable.

"**ICC**" has the meaning set forth in Section 9.3.

"**Initial Press Release**" has the meaning set forth in Section 5.3.

"**Legal Proceedings**" means Proceedings relating to the Licensed Products in various forums around the world, including those set forth on **Schedule 1**, and any other claim that either Party may have against the other as of the Effective Date, known or unknown and whether or not asserted in a Proceeding.

"**Licensed Patents**" means BioMarin's and its Affiliates' patent families worldwide having a claim covering any Licensed Product, its manufacture, use, sale, import, or otherwise, whether existing before or after the Effective Date, including, without limitation, (i) the patents and patent applications listed in **Schedule 2**; (ii) any patents or patent applications worldwide from which the patents or patent applications in **Schedule 2** claim priority; (iii) any continuations, continuations-in-part, divisionals, renewals, reissues, reexaminations, corrections, substitutions, supplementary protection certificates, extensions, or later-filed foreign counterparts of any of the foregoing; and (iv) any amended claims of any of the foregoing arising out of an inter partes review, post-grant review, or other patent proceeding.

"**Licensed Product**" means any Ascendis product, method, or service that includes the C-type natriuretic peptide known as "TransCon CNP," "navepegritide," or "YUVIWEL." For clarity, Licensed Product includes, but is not limited to, the pharmaceutical product submitted for approval under NDA 219164 and MAA Procedure EMEA/H/C/006627, as well as modifications of the same after the Effective Date. For the avoidance of doubt, the Licensed Product does not include the VOXZOGO Product.

"**Losses**" means all pending and potential claims, demands, all manner of actions, causes of action, suits, debts, liabilities, losses, damages, reasonable attorneys' fees, costs, expenses (including expert fees), judgments, settlements, interest, punitive damages and other damages or costs of whatever nature, whether known or unknown, pending or future, certain or contingent.

"**Net Sales**" means, with respect to any Licensed Product, the gross amounts actually billed, invoiced, or otherwise charged or accrued by or on behalf of a Selling Party to Third Parties for the sale or other disposition of such Licensed Product in the Territory, including all cash and the fair market value of all non-cash consideration received or receivable in connection therewith, less the following deductions to the extent attributable to the Licensed Product, actually incurred, paid, accrued or credited, in accordance with Accounting Standards:

(a) [***];

(b) [***];

(c) [***];

(d) [***];

(e) [***];

(f) [***]; and

(g) any other similar deductions, taken and consistently applied in the ordinary course of business by Ascendis in accordance with Ascendis' standard practices and applicable Accounting Standards when calculating net sales in its publicly-disclosed reports to its shareholders, as consistently applied.

Net Sales are calculated on the first sale to a Third Party in a bona fide arm's-length transaction, and will not include [***]. However, [***] will be included in Net Sales.

Net Sales will further not include [***].

All such [***] and other deductions, to the extent allocable across multiple products including a Licensed Product, shall be fairly and equitably allocated between or amongst the applicable Licensed Product and other products of Ascendis and its Affiliates and sublicensees such that such Licensed Product does not bear a disproportionate portion of such deductions.

For clarity, (i) any amounts that are deducted from Net Sales pursuant to one subsection may not be deducted pursuant to another subsection (i.e., a deduction may only be taken once) and (ii) the determination of Net Sales for all purposes under this Agreement shall be made in good faith by Ascendis in accordance with Accounting Standards and shall be consistent with Ascendis' financial statements and customary revenue recognition and reporting practices applied on a consistent basis.

If a Licensed Product is sold as part of a Combination Product containing one or more Other Active Ingredients, Net Sales shall equal [***].

Net Sales shall be calculated on a country-by-country basis based on sales or disposition of the Licensed Products within countries within the Territory or sales or dispositions of the Licensed Products made outside any such country to the extent that such sales or disposition of product are (a) [***] or (b) [***]. For clarity, [***] shall not be included in determining Net Sales for the Territory.

"**Other Active Ingredients**" means active pharmaceutical ingredients other than the C-type natriuretic peptide known as navepegritide.

"**Person**" means any individual, corporation, partnership, limited liability company, joint venture, trust, unincorporated organization, government or any agency or political subdivision thereof, or any other entity.

"**Proceeding**" means any lawsuit or any other civil or administrative proceeding, or any claim asserted in or to any court, arbitration, tribunal, agency (including, but not limited to, any government patent office), or other adjudicative body.

"**Product Liability Claims**" means any claim, demand, or lawsuit involving allegations that a manufacturing, design or warning defect, regardless of whether the claim, demand, or lawsuit sounds in negligence or strict liability, injured a patient.

"**Related Parties**" has the meaning set forth in Section 4.2. "**Royalties**" has

the meaning set forth in Section 3.1.

"**Royalty Term**" means the period beginning upon the First Commercial Sale and expiring on May 20, 2030.

"**Selling Party**" means Ascendis, its Affiliates, or any permitted sublicensees. "**Term**" has the meaning set forth in Section 8.1.

"**Territory**" means the United States (including its territories, commonwealths, districts and possessions, including the District of Columbia, and Commonwealth of Puerto Rico), the European Union, Brazil, and South Korea.

"**Third Party**" means any Person other than BioMarin, Ascendis, or their respective Affiliates.

"**VOXZOGO Product**" means the once-daily injectable vosoritide product that, as of the Effective Date, has received marketing approval by the FDA under the BioMarin NDA (as may be amended or supplemented) and the trade name "Voxzogo" for a pharmaceutical product (and any modifications, improvements or iterations thereof), containing a C-type natriuretic peptide known as "vosoritide", including any indication expansions, combination or alternative formulations thereof; provided, however, "VOXZOGO Product" does not mean or include the Licensed Product or BioMarin's product in development known as BMN 333, or any long-acting C-type natriuretic peptide products.

ARTICLE 2
LICENSE GRANT AND RESTRICTIONS

Section 2.1 **License Grant**. Subject to the terms and conditions of this Agreement, BioMarin hereby grants to Ascendis and its Affiliates under the Licensed Patents a non-exclusive, worldwide, transferable (subject to the assignment restrictions in Section 10.3), royalty-bearing (subject to Section 3.2) license, with the right to grant sublicenses (subject to Section 2.2), to research, develop, manufacture, have manufactured, use, store, have stored, sell, offer for sale, have sold, import, or otherwise commercialize any Licensed Product. The Parties acknowledge and agree that the license granted under this Section 2.1 applies retroactively to the First Commercial Sale date, in exchange for the Royalties payment under Section 3.1 during the Royalty Term, although this Agreement is entered into as of the Effective Date. The license granted under this Section 2.1 shall be irrevocable, except in the circumstance of a material breach by Ascendis or its Related Parties that is uncured within [***] days following written notice thereof from BioMarin to Ascendis (it being acknowledged and agreed by the Parties that the cure period for a material breach involving a good faith dispute about the Royalties payable hereunder shall be tolled and shall not commence until resolution through the audit procedures under Section 3.7, and Dispute resolution under Section 9.2 and arbitration under Section 9.3, in each case as applicable), in which case BioMarin shall have the right to terminate this Agreement in accordance with Article 8.

Section 2.2 **Sublicenses**. Ascendis may grant sublicenses (through multiple tiers) under the license granted in Section 2.1 with BioMarin's prior written consent, except for the authorized sublicensees identified in **Schedule 3** where BioMarin's consent is not required; provided that consent obtained for one sublicense to a Third Party shall not constitute or imply consent to any further sublicensing to any other Third Party without BioMarin's separate written consent. Each sublicense agreement (at any tier) shall (a) be consistent with and subject to the terms of this Agreement, (b) require the sublicensee to comply with all applicable obligations of

a sublicensee of Ascendis under this Agreement, (c) prohibit the sublicensee from granting further sublicenses except in accordance with this Section 2.2, and (d) include audit rights (but only for sublicenses for which rights in the Territory during the Royalty Term have been granted) and confidentiality obligations at least as protective as those set forth in this Agreement. With respect to each sublicense other than the authorized sublicensees identified in **Schedule 3**, Ascendis shall provide BioMarin with a copy of each executed sublicense agreement (which may be redacted to remove confidential and financial terms unrelated to BioMarin's rights hereunder) within [***] days of execution of such sublicense agreement. Ascendis shall remain primarily responsible for the performance of its sublicensees (at all tiers) and any act or omission of a sublicensee that would constitute a breach of this Agreement if performed by Ascendis shall be deemed a breach by Ascendis. For the avoidance of doubt, Ascendis may distribute, offer for sale, and sell the Licensed Product through wholesalers and/or distributors, and such distribution, offer, and sales shall not be deemed to constitute a sublicense; provided that a Person who is appointed by Ascendis as a distributor of Licensed Product and who is obligated or has otherwise agreed to pay any upfront payments, royalties, milestones, or substantially similar payments to Ascendis or any of its Affiliates as consideration for such appointment shall instead be deemed a sublicensee requiring BioMarin's consent under this Section 2.2.

Section 2.3 **Patent Rights**. BioMarin shall at all times retain the ownership of the BioMarin Patent Rights and control the prosecution, maintenance, and enforcement thereof. Without limiting the foregoing, BioMarin shall have the sole right, but not the obligation, to prosecute and maintain the Licensed Patents in its sole discretion. BioMarin shall have the sole right, but not the obligation, to enforce the Licensed Patents against any infringement of BioMarin Patent Rights at its sole cost and expense.

Section 2.4 **Reservation of Rights**. All rights not expressly granted to Ascendis hereunder are expressly reserved to BioMarin. Nothing in this Agreement shall be construed as granting Ascendis or its Affiliates any rights with respect to any product other than the Licensed Product.

ARTICLE 3
FINANCIALS, REPORTS, AND PAYMENTS

Section 3.1 **Royalties**. In consideration for the rights and licenses granted to Ascendis hereunder, during the Royalty Term in each country of the Territory, Ascendis shall pay to BioMarin royalties on Net Sales of each Licensed Product generated by any Selling Party in the Territory ("**Royalties**") according to the following:

(a) Ascendis shall pay BioMarin a royalty equal to twenty percent (20%) of Net Sales of Licensed Product in the United States (including its territories) on a quarterly basis; and

(b) Ascendis shall pay BioMarin a royalty equal to eighteen percent (18%) of Net Sales of Licensed Product in the European Union, Brazil, and South Korea on a quarterly basis.

Section 3.2 **Paid-Up License**. After the Royalty Term has expired, Ascendis, its Affiliates, and its sublicensees shall have a fully paid-up, irrevocable, non-exclusive, unrestricted, royalty-free license under all Licensed Patents for the Licensed Product on a worldwide basis, existing at the time of such expiration and thereafter.

Section 3.3 **Reports and Payment**. Royalties shall be payable on a quarterly basis, with Ascendis (a) delivering a royalty report (setting forth [***], and the Royalty amount payable) to BioMarin within [***] days after the end of each Calendar Quarter, and (b) paying the Royalties for such Calendar Quarter within [***] business days after delivery of such royalty report. For clarity, any royalty report delivered under this Section 3.3 will not include [***] and Ascendis shall not be obligated to disclose any competitive information, including [***]; provided that such calculations and information will be available to BioMarin's independent auditors subject to the applicable limitations set forth in Section 3.7. Notwithstanding the foregoing, the first payment of Royalties (i.e., for the period commencing on the date of First Commercial Sale in the United States) shall be due [***].

Section 3.4 **Estimated Net Sales**. Ascendis shall provide BioMarin a non-binding estimate of Net Sales, as will be reported in the royalty report for the Territory, within [***] business days after the end of each Calendar Quarter.

Section 3.5 **Payment Mechanics**. All payments under this Agreement shall be made by wire transfer of immediately available funds in United States Dollars to such bank account as BioMarin may from time to time designate in writing. If any currency conversion is required, such conversion shall be made using the average of the applicable exchange rates published in the European Central Bank (or comparable source mutually agreed by the Parties) for the last business day of each month during the applicable Calendar Quarter.

Section 3.6 **Late Payments**. Any amount due hereunder and not paid when due shall bear interest from the date due until paid at a rate equal to the lesser of (a) the prime rate as published in *The Wall Street Journal* (U.S., Eastern Edition) plus [***] and (b) the maximum rate permitted by Applicable Law.

Section 3.7 **Audit Rights**. Ascendis, its Affiliates and sublicensees (but only for sublicenses for which rights in the Territory during the Royalty Term have been granted) shall maintain accurate books and records in sufficient detail to enable the payments due hereunder to be determined for a period of at least [***] years following the end of the Calendar Quarter to which such records pertain. BioMarin shall have the right, on [***]-per-Calendar Year basis (unless a prior audit under this Section 3.7 has revealed any substantial deficiencies, in which case [***] shall be permitted) and subject to BioMarin providing at least [***] days' advance written notice, for an independent, nationally recognized certified public accounting firm auditor appointed by BioMarin to audit the determination and evaluation of Net Sales (it being acknowledged and agreed by the Parties that the auditor shall share with BioMarin only [***]), including as necessary to confirm the reconciliation report provided by Ascendis pursuant to Section 8.4; provided, however, that if the auditor has concerns regarding any specific deductions taken when reporting same to the Parties (including when reconciling accruals against actual amounts under Section 8.4), the auditor may share with BioMarin [***] (it being

acknowledged and agreed by the Parties that such auditor shall not under any circumstances, share [***] or share any documentation with BioMarin prior to Ascendis having reviewed and consented in writing to such sharing or disclosure, not to be unreasonably withheld, conditioned or delayed; provided that such auditor shall not, in any event, have the right to share or disclose any documentation or information to the extent reflecting or including any Confidential Information of Ascendis). Ascendis shall make its relevant personnel reasonably available to the auditor and shall provide the auditor with reasonable access to all relevant books, records, and supporting documentation. The Parties shall share the cost of an annual audit. Any audit conducted pursuant to this Section 3.7 will be limited to financial records necessary to verify payments due hereunder, shall cover only periods within the prior [***] months (or, with respect to any audit rights under Section 8.4, the [***]-month period following expiration of the Term or earlier termination of this Agreement), and shall not revisit any period that has been the subject of a prior audit (except to the extent necessary to confirm the reconciliation report provided by Ascendis under Section 8.4). If an audit reveals an underpayment of more than [***] for any audited period, Ascendis shall (a) pay the deficiency plus interest thereon at the rate specified in Section 3.6 from the date such payment was originally due, and (b) reimburse BioMarin for its share of the cost of such audit. If an audit reveals an overpayment, BioMarin shall credit such overpayment against future Royalty payments (or, if no further Royalties are due, promptly refund such overpayment).

Section 3.8 **Taxes**. Each Party shall be responsible for its own income taxes. If Ascendis is required by Applicable Law to withhold taxes from any payment due to BioMarin under this Agreement, Ascendis shall (a) deduct such taxes from the payment, (b) timely remit such taxes to the appropriate governmental authority, and (c) promptly provide BioMarin with an official tax receipt or other documentation sufficient to enable BioMarin to claim a foreign tax credit if applicable. Any amount so deducted or withheld and remitted to the applicable governmental authority shall be treated for all purposes of this Agreement as having been paid to BioMarin. Following such deduction or withholding, Ascendis shall be deemed to have fully satisfied the corresponding payment obligation and shall have no obligation to gross up, increase or otherwise compensate BioMarin for such deduction or withholding. The Parties shall cooperate in good faith to minimize any such withholding to the extent permitted by Applicable Law and BioMarin shall provide such certificates, forms, declarations and other documentation, as may be reasonably requested by Ascendis for purposes of obtaining any available exemption from, or reduction of, withholding taxes.

ARTICLE 4
DISMISSAL, RELEASES AND COVENANTS

Section 4.1 **Dismissal.** The Parties shall dismiss with prejudice all pending Legal Proceedings between them relating to the Licensed Product, including the actions listed in **Schedule 1** and any claims against Third Parties arising from the Legal Proceedings, no later than September 24, 2026 (or later if, under law or order of a court or administrative agency of competent jurisdiction, required to be dismissed, or dismissal is not permitted until, after September 24, 2026). Each Party shall bear its own litigation costs, fees, and other Losses for all such pending Legal Proceedings, neither Party will pay cost reimbursements under German Applicable Law, and neither Party may request, file for, or be entitled to recover any of its costs,

fees, or damages in connection therewith. Nothing in this Agreement shall be construed as an admission by either Party of liability, validity, infringement, enforceability, or damages with respect to any of the Legal Proceedings or any patents at issue therein.

Section 4.2 **Mutual Release**. Effective as of the Effective Date, each Party, on behalf of itself and its Affiliates and subsidiaries, hereby releases, acquits and forever discharges the other Party, its Affiliates and sublicensees and their respective directors, officers, employees, agents, representatives, heirs, assigns, predecessors and successors (with respect to each Party, "**Related Parties**"), from any and all Losses arising out of, derived from, predicated upon or relating to the Legal Proceedings and the subject matter thereof occurring prior to the Effective Date, including any and all claims of liability, validity, infringement, enforceability, or damages related to the Licensed Patents occurring prior to the Effective Date; provided that nothing in this Section 4.2 shall (i) release either Party from its respective obligations under this Agreement or prevent or impair the right of the releasing Party under this Section 4.2 from invoking the jurisdiction of the relevant court or governmental authority to enforce the terms and provisions of this Agreement or (ii) prevent or impair the right of the releasing Party under this Section 4.2 to bring an action or Proceeding in court or any other forum for breach of this Agreement or any representation, warranty or covenant herein.

Section 4.3 **Unknown Claims Waiver**. Each Party acknowledges that such Party may hereafter discover facts in addition to or different from those which it now knows or believes to be true with respect to the subject matter of this release, and each Party agrees that the release given herein shall be and remain in effect as a full and complete release notwithstanding any such different or additional facts. Each Party expressly waives and relinquishes, to the fullest extent permitted by law, the provisions, rights, and benefits of California Civil Code Section 1542, which provides: "A GENERAL RELEASE DOES NOT EXTEND TO CLAIMS THAT THE CREDITOR OR RELEASING PARTY DOES NOT KNOW OR SUSPECT TO EXIST IN HIS OR HER FAVOR AT THE TIME OF EXECUTING THE RELEASE AND THAT, IF KNOWN BY HIM OR HER, WOULD HAVE MATERIALLY AFFECTED HIS OR HER SETTLEMENT WITH THE DEBTOR OR RELEASED PARTY," and any similar law of any state or jurisdiction. For the avoidance of doubt, the waiver set forth in this Section 4.3 shall apply solely to claims arising out of, derived from, predicated upon, or relating to the Legal Proceedings and the Licensed Patents, and shall not extend to any other claims between the Parties.

Section 4.4 **Mutual Covenant Not to Sue**. Effective as of the Effective Date, each Party covenants not to sue, assert any claim against, or otherwise bring, maintain, or participate in any Proceeding against the other Party or any of its Related Parties, licensees, sublicensees, importers, suppliers, manufacturers, distributors, or customers: (a) in the case of BioMarin, for infringement of BioMarin Patent Rights as a result of the exploitation of the Licensed Product by or on behalf of Ascendis; or (b) in the case of Ascendis, for infringement of patent rights as a result of the exploitation of the VOXZOGO Product by or on behalf of BioMarin. This covenant not to sue applies worldwide to claims arising from or relating to the making, having made, using, selling, offering for sale, or importation of the Licensed Product or the VOXZOGO Product, as applicable (including activities in connection therewith, including the storage, holding or handling thereof), whether occurring before or after the Effective Date. Each Party

shall impose this covenant not to sue on (a) its Affiliates and (b) any Third Party to which such Party assigns, licenses, sublicenses, or otherwise transfers any rights to or under such patents that include the right to assert such patents. Notwithstanding Section 8.5 or any other provision of this Agreement to the contrary, this mutual covenant not to sue shall terminate concurrently with, and under the same conditions as, the license granted under Section 2.1, including upon termination of this Agreement by BioMarin pursuant to Section 8.2. For clarity, this covenant not to sue shall not preclude any Proceeding involving any long-acting C-type natriuretic peptide products, including BioMarin's product in development known as BMN 333 (or any successor thereof), other than the Licensed Product.

Section 4.5 **Covenant Not to Challenge.**

(a) Except to the extent required by law or order of a court or administrative agency of competent jurisdiction, and only with respect to BioMarin Patent Rights that cover the Licensed Product, Ascendis shall not, and shall cause its Affiliates not to, directly or indirectly, as long as the license under Section 2.1 and the covenant not to sue under Section 4.4 remain in full force and effect:

(i) initiate or participate in any Proceeding (including, but not limited to, any civil action, district court litigation, inter partes review, post-grant review, reexamination, opposition, or revocation) advancing any claims that any of the BioMarin Patent Rights are invalid, unenforceable, unpatentable, or not infringed; or

(ii) voluntarily assist, encourage, induce, enable, or support any other Person in connection with any Proceeding that challenges the validity, enforceability, patentability, or noninfringement of any of the BioMarin Patent Rights, including, but not limited to, by (i) cooperating with, paying for, or advocating on behalf of such challenge or Person, (ii) [***], or (iii) [***].

(b) Notwithstanding the foregoing, nothing herein shall prevent Ascendis from engaging in or undertaking any of the foregoing activities in the event that BioMarin or its Affiliate, or any licensee or assignee of the BioMarin Patent Rights, assert in a court proceeding that one of Ascendis' or its Affiliate's products (including the Licensed Product) or any regulatory approval or marketing authorization therefor infringes any of the BioMarin Patent Rights.

(c) The obligations set forth in this Section 4.4 shall survive expiration of the Term but shall not survive in the event of any termination by BioMarin pursuant to Section 8.2.

Section 4.6 **Regulatory Support and Non-Interference**.

(a) **BioMarin Regulatory Support**. BioMarin shall, and shall cause its Affiliates to: (i) waive any regulatory rights that may interfere with the regulatory approval of TransCon CNP (navepegritide) or any other Licensed Product worldwide; and (ii) upon Ascendis' request, promptly provide any regulatory waivers, consents, or authorizations as requested by Ascendis to facilitate such approvals and the commercialization of Licensed

Products worldwide, and in any event within [***] business days of Ascendis' written request therefor.

(b) **Ascendis Regulatory Support**. Ascendis shall, and shall cause its Affiliates to: (i) waive any regulatory rights that may interfere with the regulatory approval of the VOXZOGO Product worldwide; and (ii) upon BioMarin's request, promptly provide any regulatory waivers, consents, or authorizations as requested by BioMarin to facilitate such approvals and the commercialization of the VOXZOGO Product worldwide, and in any event within [***] business days of BioMarin's written request therefor.

(c) **Non-Interference Covenant**. Neither Party shall, directly or indirectly: (i) seek to influence any regulatory authority to deny, delay, or condition approval for the other Party's product (i.e., TransCon CNP (navepegritide) or other Licensed Product with respect to Ascendis, and VOXZOGO Product with respect to BioMarin) covered by this Section 4.6; (ii) interfere with the other Party's patent prosecution or enforcement activities; or (iii) take any other action intended to impede the other Party's commercialization (in the ordinary course of business) of such TransCon CNP (navepegritide) or Licensed Product, or VOXZOGO Product in the Territory, it being acknowledged and agreed by the Parties that such obligation not to impede shall not mean, or be deemed to mean, that Ascendis or BioMarin cannot take any other action that is pro-competitive or otherwise supporting its product in comparison to other products, including Ascendis' TransCon CNP (navepegritide) or Licensed Product or BioMarin's VOXZOGO Product, in each case in compliance with Applicable Law.

Section 4.7 **Compliance with Applicable Law**. Each Party shall, and shall cause its Affiliates and sublicensees to, comply with all Applicable Law in connection with the development, manufacture, use, storage, importation, marketing, distribution, sale, offer for sale, and other commercialization of the Licensed Product or the VOXZOGO Product, as applicable, in the Territory and in the performance of its obligations and exercise of its rights under this Agreement. Each Party shall obtain and maintain all permits, licenses, registrations, and regulatory approvals required under Applicable Law for such activities.

ARTICLE 5 CONFIDENTIALITY AND PUBLICITY

Section 5.1 **Confidential Information**. Each Party agrees that, subject to the information that may be disclosed under Section 5.3, (a) the terms of this Agreement, (b) all information relating to the negotiation and execution of this Agreement, (c) payment terms and royalty amounts under this Agreement, or (d) any other nonpublic information disclosed (i) by one Party to the other Party in connection with this Agreement or (ii) under a protective order with respect to any Legal Proceedings prior to the Effective Date (collectively, "**Confidential Information**") shall be kept confidential and shall not be disclosed to any Third Party without the prior written consent of the other Party, except (i) to such Party's Affiliates, directors, officers, employees, agents, legal advisors, financial advisors, and accountants who have a need to know and who are bound by obligations of confidentiality at least as restrictive as those set forth herein, (ii) as required by Applicable Law (including applicable securities laws and related public disclosures in connection therewith), (iii) to a bona fide potential or actual acquirer, merger partner, investor,

or lender (or their respective advisors) in connection with a potential transaction, subject to customary confidentiality obligations, (iv) in connection with filings with the U.S. Federal Trade Commission, the U.S. Department of Justice, or any other governmental authority, or (v) as otherwise permitted by this Article 5.

Section 5.2 **Standard Exceptions**. The obligations of confidentiality under this Article 5 shall not apply to information that (a) is or becomes generally available to the public other than as a result of a breach of this Agreement, (b) was known to the receiving Party prior to its disclosure hereunder, as evidenced by written records, (c) is independently developed by the receiving Party without use of or reference to the Confidential Information of the disclosing Party, as evidenced by written records, or (d) is disclosed to the receiving Party by a Third Party having no obligation of confidentiality to the disclosing Party with respect to such information. For the avoidance of doubt, the Parties may disclose the Royalty amounts actually paid or received (not estimates) in compliance with applicable securities laws.

Section 5.3 **Press Release**. Each Party acknowledges that it issued a press release on August 31, 2026 in connection with the Binding Term Sheet (the "**Initial Press Release**"). Neither Party shall issue any further press release or public statement regarding this Agreement or the transactions contemplated hereby without the prior written consent of the other Party; provided, however, that either Party may, without such consent, issue a press release or public statement that is limited to the information set forth in its Initial Press Release or that repeats information contained in its Initial Press Release, or that discloses the fact of settlement, the grant of the license and covenant not to sue, the waiver of regulatory exclusivities, the Territory, the Royalty Term, and the Royalty rates and applicable countries. Any disclosure made in accordance with the preceding sentence shall not constitute a breach of this Article 5.

ARTICLE 6
REPRESENTATIONS, WARRANTIES, AND COVENANTS

Section 6.1 **Mutual Representations and Warranties**. Each Party hereby represents and warrants to the other Party as of the Effective Date that: (a) such Party is a corporation or other entity duly organized, validly existing, and in good standing under the laws of the jurisdiction of its organization; (b) the execution, delivery, and performance of this Agreement by such Party have been duly authorized by all necessary corporate or other action on the part of such Party; (c) this Agreement has been duly executed and delivered by such Party and constitutes a legal, valid, and binding obligation of such Party, enforceable against such Party in accordance with its terms; (d) such Party's execution, delivery, and performance of this Agreement do not violate, conflict with, or result in a breach of any provision of its organizational documents or any Applicable Law, order, or agreement to which it is a party; and (e) it has the right and authority to settle the Legal Proceedings.

Section 6.2 **BioMarin Representations and Covenant Regarding License.** In addition to Section 6.1, BioMarin represents and warrants to Ascendis that, as of the Effective Date:

(a) Except to the extent of any specific allegations raised by Ascendis prior to the Effective Date as to ownership of the BioMarin Patent Rights in the actions listed in

Schedule 1, BioMarin is the owner of the Licensed Patents (subject to any security interests) and has the right to grant to Ascendis the licenses granted hereunder;

(b) BioMarin has not previously assigned, transferred, conveyed, or granted any license (or sublicense) or other rights under the BioMarin Patent Rights that would conflict with or limit the scope of any of the rights or licenses granted to Ascendis under this Agreement; and

(c) BioMarin is not under any obligation, contractual or otherwise, to any Person that conflicts with or is inconsistent in any material respect with the terms of this Agreement.

Section 6.3 **Ascendis Representation**. Ascendis represents and warrants to BioMarin, as of the Effective Date, that the sublicense agreements entered into with the authorized sublicensees identified in **Schedule 3** do not and will not include any countries in the Territory.

Section 6.4 **Ascendis Representations and Covenant Regarding License**. In addition to Section 6.1, Ascendis represents and warrants to BioMarin that, as of the Effective Date, Ascendis has not assigned, transferred, or granted, and will not assign, transfer or grant, any license (or sublicense) or other rights under the BioMarin Patent Rights that would conflict with or limit the scope of any of the rights or licenses granted to Ascendis under this Agreement or prevent Ascendis from performing its obligations under this Agreement.

Section 6.5 **Disclaimer**. EXCEPT AS EXPRESSLY PROVIDED IN THIS AGREEMENT, NEITHER PARTY MAKES ANY REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, EITHER IN FACT OR BY OPERATION OF APPLICABLE LAW, AND EACH PARTY HEREBY EXPRESSLY DISCLAIMS SUCH WARRANTIES.

ARTICLE 7 INDEMNIFICATION

Section 7.1 **Indemnification by Ascendis**. Ascendis shall indemnify, defend, and hold harmless BioMarin, its Affiliates, and their respective directors, officers, employees, and agents (collectively, "**BioMarin Indemnitees**") from and against any and all Losses to the extent arising out of or resulting from any Third Party claim, suit, action, or Proceeding to the extent arising or resulting from (a) Ascendis' development, manufacture, use, importation, sale, offer for sale, and/or commercialization of the Licensed Product, including any Product Liability Claims to the extent involving a Licensed Product, but excluding any Losses to the extent arising from BioMarin's own development, manufacture, use, or commercialization of any product (including the VOXZOGO Product), or (b) Ascendis' exercise of the license granted hereunder, in each case (clause (a) or (b)), except to the extent such claims arise or result from BioMarin's material breach of this Agreement, gross negligence, recklessness, willful misconduct or fraud, or non-compliance with Applicable Law.

Section 7.2 **Indemnification by BioMarin**. BioMarin shall indemnify, defend, and hold harmless Ascendis, its Affiliates, and their respective directors, officers, employees, and agents (collectively, "**Ascendis Indemnitees**") from and against any and all Losses to the extent arising out of or resulting from any Third Party claim, suit, action, or Proceeding to the extent arising or

resulting from BioMarin's development, manufacture, use, importation, sale, offer for sale, and/or commercialization of the VOXZOGO Product, including any Product Liability Claims to the extent involving such VOXZOGO Product, but excluding any Losses to the extent arising from Ascendis' own development, manufacture, use, or commercialization of any Licensed Product, in each case, except to the extent such claims arise or result from Ascendis' material breach of this Agreement, gross negligence, recklessness, willful misconduct or fraud, or non-compliance with Applicable Law.

Section 7.3 **Indemnification Procedures.** The indemnification obligations set forth in Sections 7.1 and 7.2 shall be contingent upon the BioMarin Indemnitee or the Ascendis Indemnitee, as applicable: (a) notifying the other Party of a claim, demand, or suit within [***] days of receipt thereof; provided that the BioMarin Indemnitee's or Ascendis Indemnitee's (as applicable) failure or delay in providing such notice shall not relieve the indemnifying Party of its indemnification obligations hereunder; (b) allowing the indemnifying Party the right to assume direction and control of the defense of any such claim, demand, or suit; (c) cooperating with such indemnifying Party in the defense of such claim, demand, or suit at such indemnifying Party's expense; and (d) agreeing not to settle or compromise any claim, demand, or suit without the indemnifying Party's prior written authorization. The BioMarin Indemnitee or Ascendis Indemnitee (as applicable) shall have the right to participate in the defense of any such claim, demand, or suit referred to in this Section 7.3 utilizing attorneys of its choice, at its own expense (for any reason, including if representation of the BioMarin Indemnitee or Ascendis Indemnitee, as applicable, by the counsel retained by the indemnifying Party would be inappropriate due to actual or potential conflicts or differing interests between such BioMarin Indemnitee or Ascendis Indemnitee and such indemnifying Party), provided that the indemnifying Party shall have full authority and control to handle any such claim, demand, or suit. The indemnifying Party will not settle any such proceeding under terms that would (a) result in an admission of invalidity or unenforceability of any BioMarin Patent Rights, (b) result in the indemnified Party incurring financial liability, or (c) require an admission of liability or fault on the part of the indemnified Party, in each case, without the indemnified Party's prior written consent.

Section 7.4 **Insurance**. Each Party shall obtain and maintain, at its sole cost and expense, commercial general liability insurance and product liability insurance with limits of not less than [***] per occurrence and [***] in the aggregate, covering claims arising out of the development, manufacture, use, sale, offer for sale, importation, and commercialization of the Licensed Product or the VOXZOGO Product, as applicable. Such insurance shall (a) be obtained from insurers with an A.M. Best rating of [***], (b) name the other Party as an additional insured, (c) include a waiver of subrogation in favor of such other Party, and (d) require the insurer to provide such other Party with at least [***] days' prior written notice of any cancellation, non-renewal, or material change in coverage. Upon a Party's request, the other Party shall provide such Party with certificates of insurance evidencing such coverage. Such insuring Party shall maintain such insurance for so long as it is commercializing the Licensed Product or the VOXZOGO Product, as applicable, and for a period of [***] years thereafter.

Section 7.5 **Limitation of Liability.** IN NO EVENT SHALL EITHER PARTY BE LIABLE TO THE OTHER PARTY FOR ANY INDIRECT, INCIDENTAL, SPECIAL, CONSEQUENTIAL, OR PUNITIVE DAMAGES ARISING OUT OF OR RELATED TO THIS

AGREEMENT, REGARDLESS OF WHETHER SUCH DAMAGES ARE BASED ON CONTRACT, TORT, STRICT LIABILITY, OR ANY OTHER THEORY, EVEN IF SUCH PARTY HAS BEEN ADVISED OF THE POSSIBILITY OF SUCH DAMAGES; PROVIDED THAT THIS LIMITATION SHALL NOT APPLY TO (A) ASCENDIS' OR BIOMARIN'S INDEMNIFICATION OBLIGATIONS UNDER THIS ARTICLE 7, (B) A PARTY'S BREACH OF ARTICLE 5 (CONFIDENTIALITY AND PUBLICITY), (C) A PARTY'S GROSS NEGLIGENCE, WILLFUL MISCONDUCT OR FRAUD, OR (D) ASCENDIS' BREACH OF THE COVENANT NOT TO CHALLENGE UNDER SECTION 4.5.

ARTICLE 8
TERM AND TERMINATION

Section 8.1 **Term.** This Agreement shall become effective on the Effective Date and, unless earlier terminated in accordance with this Article 8, shall remain in full force and effect until the expiration of the Royalty Term (the "**Term**"); provided that certain provisions shall survive as set forth in Section 8.5.

Section 8.2 **Termination for Material Breach**. The license granted under this Agreement is irrevocable except that, in the event of a material breach by Ascendis or its Related Parties of this Agreement, BioMarin may terminate this Agreement upon written notice to Ascendis if such breach remains uncured for [***] days following Ascendis' receipt of written notice thereof specifying the nature of the breach in reasonable detail (it being acknowledged and agreed by the Parties that the cure period for a material breach involving a good faith dispute about the Royalties payable hereunder shall be tolled and shall not commence until resolution through the audit procedures under Section 3.7, and Dispute resolution under Section 9.2 and arbitration under Section 9.3, in each case as applicable). Termination under this Section 8.2 shall be the sole termination right available to either Party under this Agreement.

Section 8.3 **Effects of Expiration or Termination**.

(a) Upon expiration of the Royalty Term, the license granted under Section 2.1 shall convert to a fully paid-up, irrevocable, non-exclusive, unrestricted, royalty-free license as set forth in Section 3.2.

(b) Upon termination of this Agreement by BioMarin pursuant to Section 8.2 (i) all rights and licenses granted to Ascendis hereunder shall terminate; (ii) the covenant not to sue set forth in Section 4.3 shall survive as provided therein; (iii) all sublicenses granted by Ascendis shall terminate, unless BioMarin elects in writing within [***] days to assume any such sublicense as a direct license between BioMarin and the applicable sublicensee on the same terms and conditions as such sublicenses were in effect immediately prior to such termination; and (iv) Ascendis shall pay all Royalties accrued through the date of termination within [***] days thereof.

(c) Termination shall not relieve either Party of any obligation accrued prior to such termination.

Section 8.4 **Contract-End Reconciliation**. Within [***] year following the expiration of the Term or termination of this Agreement, Ascendis will provide a final reconciliation report

substantially consistent with the royalty reports provided by Ascendis under Section 3.3 of all accrued calculations and deductions made pursuant to the definition of Net Sales and of all amounts payable or processed hereunder. The reconciliation shall be based on all actual amounts known through the date that is [***] months following the applicable expiration or termination date versus prior accruals or estimates. No further reconciliations shall be made thereafter. BioMarin shall have the right, at its sole cost and expense and in accordance with Section 3.7, for an independent, nationally recognized certified public accounting firm to audit such reconciliation report. If any reconciliation under this Section 8.4 shows an underpayment or an overpayment, and (i) Ascendis agrees, then Ascendis or BioMarin, as applicable, shall pay the amount of the difference to the other Party within [***] days of the date of delivery of the reconciliation report or (ii) Ascendis disputes such reconciliation, then such dispute shall be referred to Dispute resolution under Section 9.2 and arbitration under Section 9.3, as applicable.

Section 8.5 **Survival.** The following provisions shall survive any expiration or termination of this Agreement: Article 1 (Definitions), Section 3.2 (Paid-Up License) (only upon expiration, not termination), Section 3.7 (Audit Rights) (for a period of [***] years following expiration or termination), Section 4.1, Section 4.2 (only upon expiration, not termination), Section 4.3 (only upon expiration, not termination), Section 4.5 (only upon expiration, not termination), Article 5 (Confidentiality and Publicity), Article 6 (Representations, Warranties, and Covenants), Article 7 (Indemnification), Section 8.4 (Contract-End Reconciliation), this Section 8.5 (Survival), Article 9 (Governing Law and Dispute Resolution), and Article 10 (General Provisions).

Section 8.6 **Bankruptcy Protection.** All rights and licenses granted under or pursuant to this Agreement are, and shall otherwise be deemed to be, for purposes of Section 365(n) of the U.S. Bankruptcy Code (11 U.S.C. Section 365(n)), licenses of "intellectual property" as defined under Section 101 of the Bankruptcy Code. Each Party shall retain and may fully exercise all of its respective rights and elections under the Bankruptcy Code.

ARTICLE 9
GOVERNING LAW AND DISPUTE RESOLUTION

Section 9.1 **Governing Law.** This Agreement shall be governed by and construed in accordance with the laws of the State of New York, without giving effect to any choice of law or conflict of law provision or rule that would cause the application of the laws of any other jurisdiction.

Section 9.2 **Executive Escalation**. Any dispute, controversy or claim arising out of or relating to this Agreement (a "**Dispute**") shall first be submitted to the Chief Executive Officers (or their designees) of each Party by written notice from either Party to the other specifying the terms of such Dispute in reasonable detail. Within [***] days of receipt of such notice, the Chief Executive Officers (or their designees) shall meet in person or by telephone for the purpose of resolving such Dispute. They shall negotiate in good faith for a period of up to [***] days in an

effort to resolve the Dispute. If the Dispute is not resolved within such period, either Party may proceed to arbitration pursuant to Section 9.3.

Section 9.3 **Arbitration.** If a Dispute is not resolved through executive escalation pursuant to Section 9.2, any controversy or claim arising out of or relating to this Agreement, or the breach thereof, shall be settled by arbitration administered by the International Chamber of Commerce ("**ICC**") in accordance with the ICC Rules of Arbitration in effect at the time of the arbitration. Claims shall be heard by a single arbitrator. The arbitration shall be governed by the laws of the State of New York. The place of arbitration shall be New York, New York. The arbitration proceedings, including any filings, evidence, testimony, and the arbitrator's award, shall be kept confidential by the Parties and the arbitrator, except to the extent disclosure is required by Applicable Law or is necessary to confirm, vacate, or enforce the award. Judgment on the award rendered by the arbitrator may be entered in any court having jurisdiction thereof.

Section 9.4 **Equitable Relief.** Each Party acknowledges that any breach of this Agreement may cause the other Party irreparable harm for which monetary damages would be inadequate. Accordingly, and notwithstanding Section 9.2 and Section 9.3, either Party may apply to any court of competent jurisdiction for a temporary restraining order, preliminary injunction, specific performance, or other equitable relief, without first submitting the matter to executive escalation or arbitration, without proving actual damages, and without posting any bond, in addition to any other remedies available at law or in equity. For the avoidance of doubt, this Section 9.4 shall not be used to circumvent the arbitration framework set forth in Section 9.3 for the resolution of substantive disputes regarding patent rights, Royalty obligations, or other monetary claims under this Agreement.

ARTICLE 10 GENERAL PROVISIONS

Section 10.1 **Entire Agreement.** This Agreement, including the Schedules and Exhibits attached hereto, constitutes the entire agreement between the Parties with respect to the subject matter hereof and supersedes any and all prior or contemporaneous agreements and understandings, whether written or oral, between the Parties with respect to the subject matter hereof, including the Binding Term Sheet by and between BioMarin and Ascendis.

Section 10.2 **Amendments.** This Agreement may not be amended or modified except by an instrument in writing signed by authorized representatives of both Parties.

Section 10.3 **Assignment**. This Agreement shall be binding upon and inure to the benefit of each Party's respective successors and permitted assigns. Neither Party may assign this Agreement without the prior written consent of the other Party, such consent not to be unreasonably withheld, delayed, or conditioned; provided, however, that, without such consent, each Party may assign this Agreement to (i) an Affiliate of such Party or (ii) any entity that acquires substantially all of that portion of such Party's assets or business to which this Agreement relates, or in connection with a merger, reorganization, or change of control. Any purported assignment in violation of this Section 10.3 shall be null and void. No assignment shall relieve the assigning Party of any of its obligations hereunder.

Section 10.4 **Notices.** All notices required or permitted under this Agreement shall be in writing and shall be deemed given when delivered personally, sent by nationally recognized overnight courier, or sent by email (with confirmation of receipt) to the addresses set forth below or to such other address as either Party may designate by written notice to the other Party:

If to BioMarin:

BioMarin Pharmaceutical Inc. 770 Lindaro Street
San Rafael, CA 94901 Attention: Chief Legal Officer

Email: [***]

With a copy (which shall not constitute notice) to:

Paul Hastings LLP 200 Park Avenue
New York, NY 10166
Attention: [***]
Email: [***]

If to Ascendis:

Ascendis Pharma A/S
Tuborg Boulevard 12
DK-2900 Hellerup
Denmark
Attention: President and CEO
[***]

With a copy (which shall not constitute notice) to:

Ascendis Pharma A/S
Tuborg Boulevard 12
DK-2900 Hellerup
Denmark
Attention: Chief Legal Officer
[***]

Section 10.5 **No Waiver.** The failure of either Party to enforce at any time any provision of this Agreement shall not be construed as a waiver of such provision or the right of such Party thereafter to enforce such provision.

Section 10.6 **Severability.** If any provision of this Agreement is held to be invalid, illegal, or unenforceable, the remaining provisions shall continue in full force and effect, and the Parties shall negotiate in good faith a replacement provision that is valid and enforceable and that most closely approximates the intent and economic effect of the invalid provision.

Section 10.7 **Counterparts.** This Agreement may be executed in one or more counterparts, each of which shall be deemed an original but all of which together shall constitute one and the same Agreement. Signatures transmitted by electronic means shall be deemed original signatures.

Section 10.8 **No Third-Party Beneficiaries.** Except as expressly provided herein, including the beneficiaries of the release in Section 4.2 and the covenant not to sue in Section 4.3, each of whom is an intended third-party beneficiary of, and may enforce, such provision, nothing in this Agreement is intended to or shall confer upon any Third Party any legal or equitable right, benefit, or remedy of any nature whatsoever under or by reason of this Agreement.

Section 10.9 **Headings.** The headings in this Agreement are for convenience of reference only and shall not affect the meaning or interpretation of this Agreement.

Section 10.10 **Independent Contractors.** The Parties are independent contractors. Nothing in this Agreement shall be construed to create a partnership, joint venture, agency, or employment relationship between the Parties.

Section 10.11 **Further Assurances.** Each Party agrees to execute and deliver such additional documents and instruments and to perform such additional acts as may be reasonably necessary to effectuate the purposes and intent of this Agreement.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the Parties have executed this Agreement as of the Effective Date.

BIOMARIN PHARMACEUTICAL INC.

By: /s/ G. Eric Davis

Name: G. Eric Davis Title: Chief Legal Officer

ASCENDIS PHARMA A/S

By: /s/ Jan Møller Mikkelsen
Name: Jan Møller Mikkelsen

Title: Authorized Representative

ASCENDIS PHARMA A/S

By: /s/ Michael Wolff Jensen

Name: Michael Wolff Jensen

Title: Authorized Representative

SCHEDULE 1

Legal Proceedings

[***]

SCHEDULE 2

Listed Patents

[***]

SCHEDULE 3

Authorized Sublicensees

[***]

Exhibit A

TransCon CNP," "navepegritide," or "YUVIWEL" Sequence Listing:

[***]

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: November 3, 2026

/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: November 3, 2026

/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, 2026, 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: November 3, 2026

/s/ BRIAN R. MUELLER

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

Date: November 3, 2026

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.