

# Third Quarter 2024 Earnings

*October 29, 2024*

# Table of Contents

- Business Overview & Highlights
- Q3 & YTD 2024 Key Financial Metrics
- Guidance & Outlook
- Non-GAAP Reconciliations

# Forward-Looking Statements

This non-confidential presentation and the associated conference call and webcast contain forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: future financial performance, including the expectations of Total Revenues, Non-GAAP Operating Margin percentage, and Non-GAAP Diluted EPS for the full-year 2024 and the underlying drivers of those results, such as the revenue opportunity represented by treatments for skeletal conditions, as well as the expectations relating to operating cash flow per year starting in 2027 and BioMarin's compound annual growth rate (CAGR) for total revenues through 2034; BioMarin's new corporate strategy and belief that such strategy will build on BioMarin's legacy of innovation to deliver even greater value to the patients it serves around the world and position BioMarin for significant growth; BioMarin's commitment to expand profitability while investing in innovation; BioMarin's ability to accelerate the VOXZOGO opportunity; the continued growth potential of BioMarin's Enzyme Therapies portfolio; the anticipated benefits from its organizational redesign efforts; BioMarin's updated strategy for ROCTAVIAN and its anticipated benefits; the timing of orders for commercial products; BioMarin's ability to meet product demand; the timing of BioMarin's clinical development and commercial prospects, including announcements of data from clinical studies and trials; the clinical development and commercialization of BioMarin's product candidates and commercial products, including (i) the potential to leverage VOXZOGO in conditions beyond achondroplasia, such as hypochondroplasia, idiopathic short stature, Noonan syndrome, Turner syndrome, SHOX deficiency and other genetic short stature pathway conditions, (ii) the expected completion of enrollment for the pivotal study of VOXZOGO in hypochondroplasia in the first half of 2025, (iii) the expected expansion of VOXZOGO in the U.S. and BioMarin's ability to enable VOXZOGO access into more than 20 additional countries by 2027, (iv) BioMarin's expectation to initiate the first-in-human study for BMN 333 in early 2025, (v) BioMarin's expectation to receive initial proof-of-concept data regarding BMN 351 in 2025, (vi) BioMarin's expectation to start dosing the multiple-ascending dose phase of the study for BMN 349 by the end of the year, (vii) BioMarin's expectation to receive data from the phase 3 study with PALYNZIQ in adolescents ages 12-17 in 2025 to support a potential U.S. Supplemental Biologics License Application in the second half of the year, (viii) BioMarin's expectations to submit an IND for BMN 390 in the second half of 2025, and (ix) BioMarin's expectation to potentially submit an IND for BMN 370 during the second half of 2025; the expected benefits and availability of BioMarin's commercial products and product candidates; and potential growth opportunities and trends, including BioMarin's plans to drive growth of its Enzyme Therapies across the 80 countries in which they are available. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others, those factors detailed in BioMarin's press release issued October 29, 2024 and BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's Annual Report on Form 10-K for the year ended December 31, 2023 as such factors may be updated by any subsequent reports including BioMarin's Quarterly Report on Form 10-Q for the quarter ended June 30, 2024. You are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

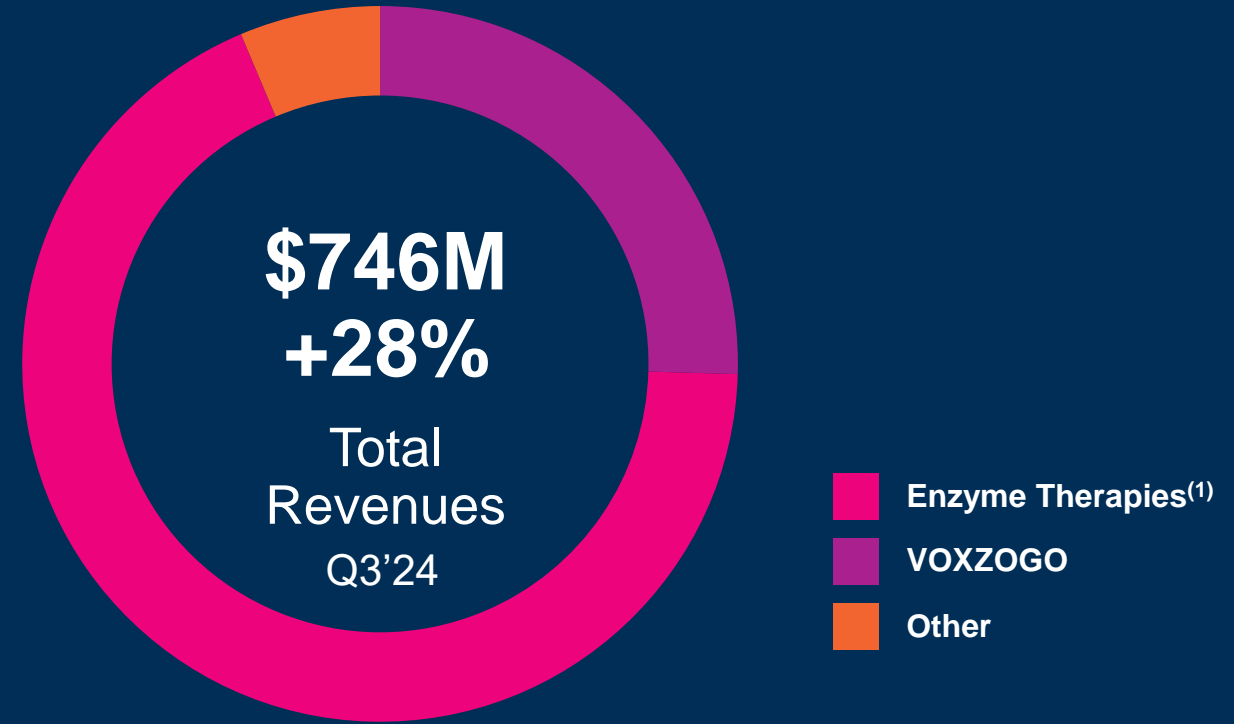
# Non-GAAP Financial Measures

This presentation includes both GAAP information and Non-GAAP information. Non-GAAP Income is defined by the company as GAAP Net Income excluding amortization of intangible assets, stock-based compensation expense, and, in certain periods, certain other specified items, as detailed below when applicable. The company also includes a Non-GAAP adjustment for the estimated tax impact of the reconciling items. Non-GAAP Operating Margin percentage is defined by the company as GAAP Income from Operations, excluding amortization of intangible assets, stock-based compensation expense, and, in certain periods, certain other specified items, divided by GAAP Total Revenues. Non-GAAP Diluted EPS is defined by the company as Non-GAAP Income divided by Non-GAAP Weighted-Average Diluted Shares Outstanding. Non-GAAP Weighted-Average Diluted Shares Outstanding is defined by the company as GAAP Weighted-Average Diluted Shares Outstanding, adjusted to include any common shares issuable under the company's equity plans and convertible debt in periods when they are dilutive under Non-GAAP.

BioMarin regularly uses both GAAP and Non-GAAP results and expectations internally to assess its financial operating performance and evaluate key business decisions related to its principal business activities: the discovery, development, manufacture, marketing and sale of innovative biologic therapies. Because Non-GAAP Income, Non-GAAP Operating Margin percentage, Non-GAAP Diluted EPS, and Non-GAAP Weighted-Average Diluted Shares Outstanding are important internal measurements for BioMarin, the company believes that providing this information in conjunction with BioMarin's GAAP information enhances investors' and analysts' ability to meaningfully compare the company's results from period to period and to its forward-looking guidance, and to identify operating trends in the company's principal business. BioMarin also uses Non-GAAP Income internally to understand, manage and evaluate its business and to make operating decisions, and compensation of executives is based in part on this measure.

Non-GAAP Income and its components are not meant to be considered in isolation or as a substitute for, or superior to, comparable GAAP measures and should be read in conjunction with the consolidated financial information prepared in accordance with GAAP. Investors should note that the Non-GAAP information is not prepared under any comprehensive set of accounting rules or principles and does not reflect all of the amounts associated with the company's results of operations as determined in accordance with GAAP. Investors should also note that these Non-GAAP financial measures have no standardized meaning prescribed by GAAP and, therefore, have limits in their usefulness to investors. In addition, from time to time in the future there may be other items that the company may exclude for purposes of its Non-GAAP financial measures; likewise, the company may in the future cease to exclude items that it has historically excluded for purposes of its Non-GAAP financial measures. Because of the non-standardized definitions, the Non-GAAP financial measure as used by BioMarin in this presentation may be calculated differently from, and therefore may not be directly comparable to, similarly titled measures used by other companies.

During Q3'24, BioMarin executed on its **new corporate strategy**, delivering **strong revenue growth and profitability expansion**; **full year guidance raised at mid-point**



**\$190M**  
**+54%**

VOXZOGO Revenues

**\$509M**  
**+27%**

Enzyme Therapies Revenues

**27.7%**  
**+11.5 ppts**

Non-GAAP Operating Margin<sup>(2)</sup>

**\$0.91**  
**+98%**

Non-GAAP Diluted Earnings per Share<sup>(2)</sup>

All figures represent the third quarter 2024; growth rates are compared to third quarter 2023

(1) Enzyme Therapies include VIMIZIM, NAGLAZYME, PALYNZIQ, BRINEURA, and ALDURAZYME

(2) Refer to slide 4 and the appendix to this presentation for the definitions of Non-GAAP Operating Margin percentage and Non-GAAP Diluted Earnings per Share and reconciliations to the comparable information reported under U.S. GAAP

# Execution on New Corporate Strategy in the Third Quarter 2024

## INNOVATION

- Phase 3 registration-enabling study with **VOXZOGO** in hypochondroplasia advanced and expected to complete enrollment in 1H'25
- Four additional new indications with **VOXZOGO** underway and **BMN 333** on track for initiation of first-in-human study in early 2025
- **BMN 351**: Completed enrollment in first dose cohort and **proof-of-concept data expected in 2025**

## GROWTH

- **VOXZOGO** Q3 revenues increased **54% Y/Y**, driven by patient growth across all regions
- **Over 3,800** children on therapy as of quarter-end; **majority** of U.S. starts were for **children under the age of 5 years**; expanding **VOXZOGO** access into **more than 20 additional countries by 2027**
- **Enzyme Therapies** Q3 revenues grew **27% Y/Y**, with the majority achieving **double-digit growth** within the quarter

## VALUE COMMITMENT

- **Leveraged revenue growth and focused operational excellence** drove profitability expansion; Q3 and year-to-date Non-GAAP diluted EPS **growing more than three times faster than revenue**
- **FY'24 guidance raised at the midpoint** for Total Revenues, Non-GAAP Operating Margin %, and Non-GAAP Diluted EPS
- **Reaffirmed long-term guidance and outlook** previously provided on September 4<sup>th</sup>

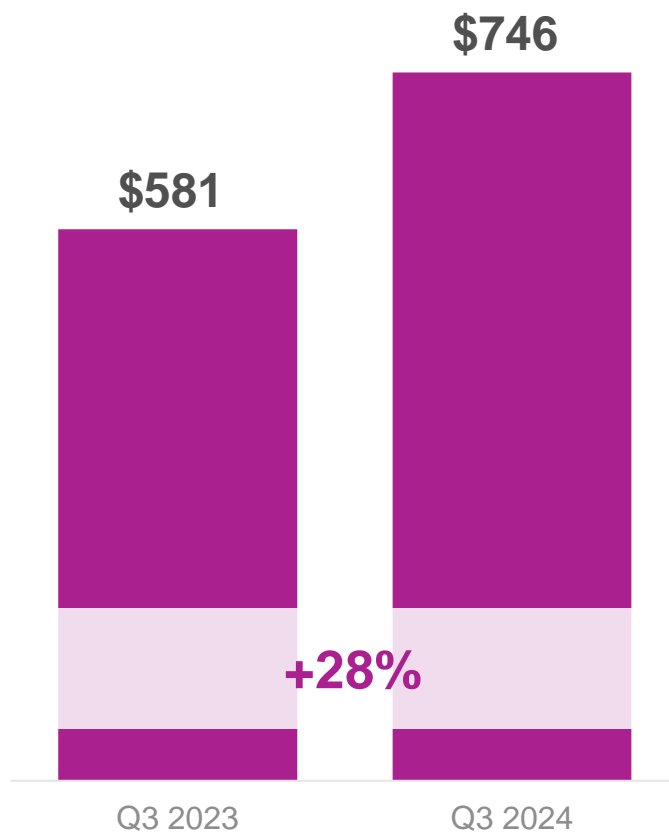
(1) Refer to slide 4 of this presentation for the definitions of Non-GAAP Diluted EPS and Non-GAAP Operating Margin percentage.

(2) Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding.

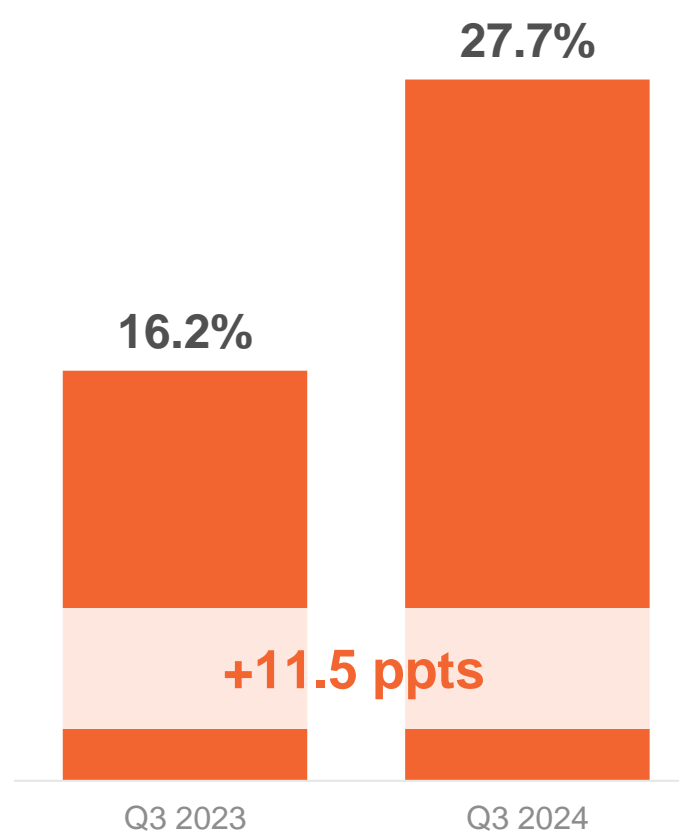
# Third Quarter 2024 Key Financial Metrics

(In millions, except share and percentage data)

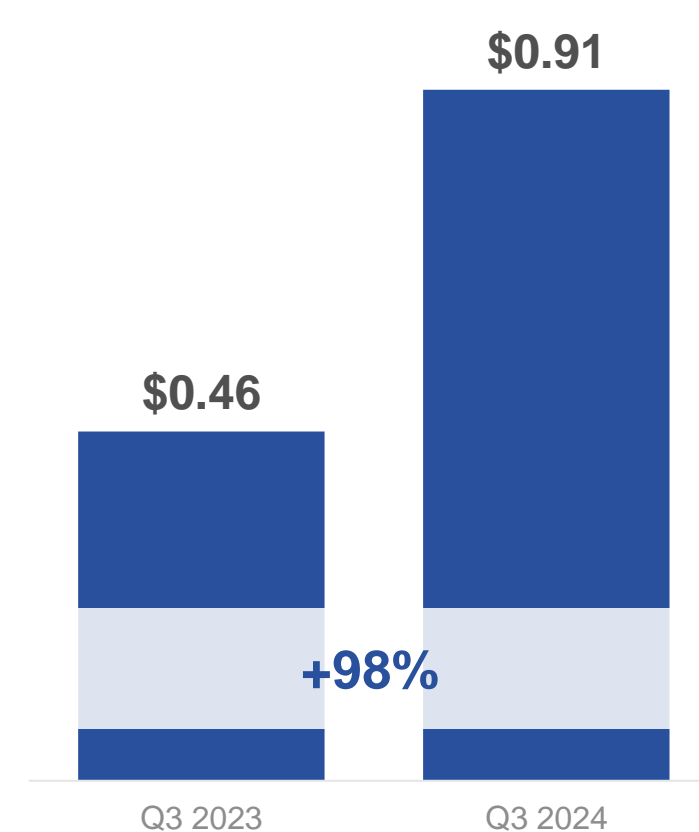
## Total Revenue Growth



## Non-GAAP Operating Margin %<sup>(1)</sup>



## Non-GAAP Diluted EPS<sup>(1)</sup>

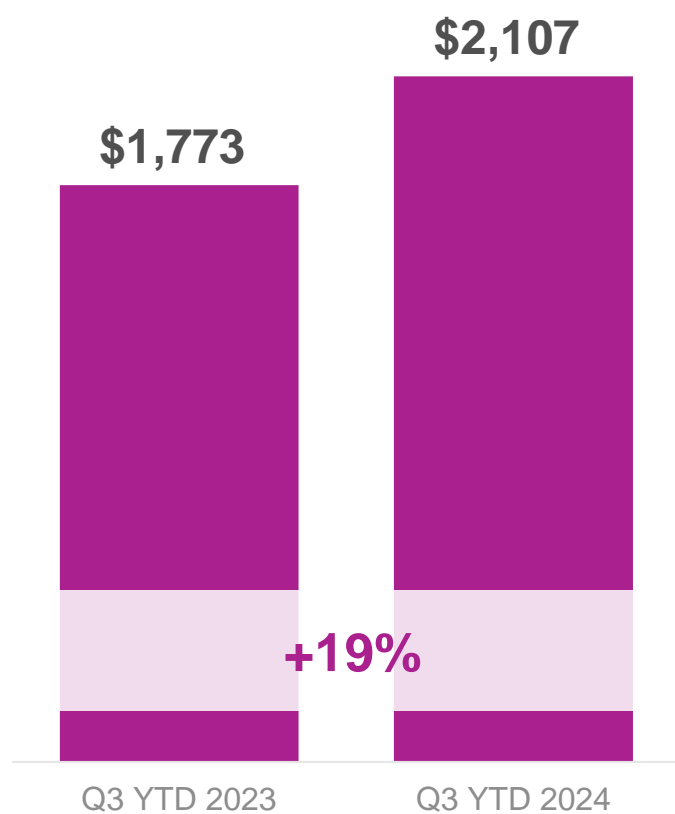


(1) Refer to slide 4 and the appendix to this presentation for the definitions of Non-GAAP Operating Margin percentage and Non-GAAP Diluted EPS and reconciliations to the comparable information reported under U.S. GAAP.

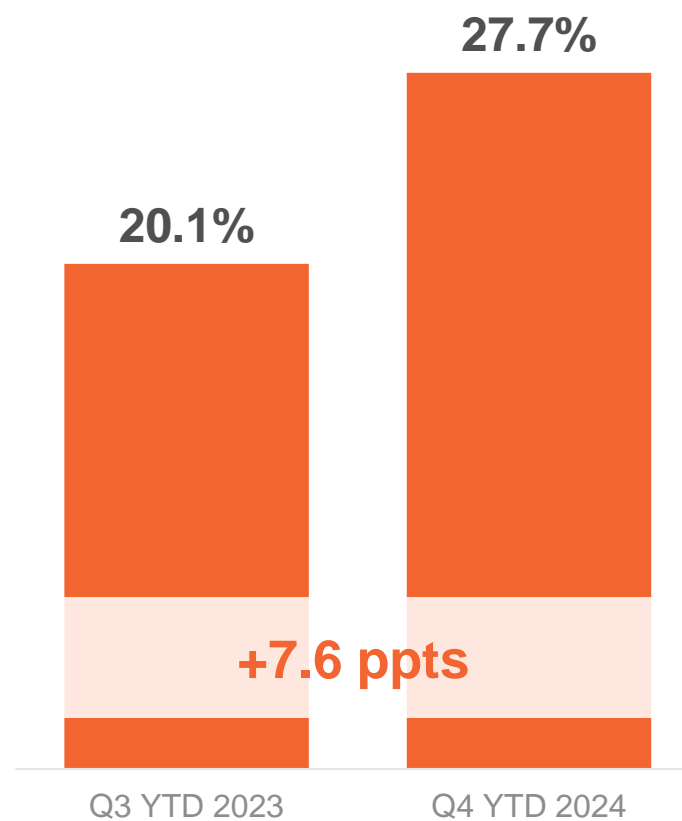
# Third Quarter Year-to-date 2024 Key Financial Metrics

(In millions, except share and percentage data)

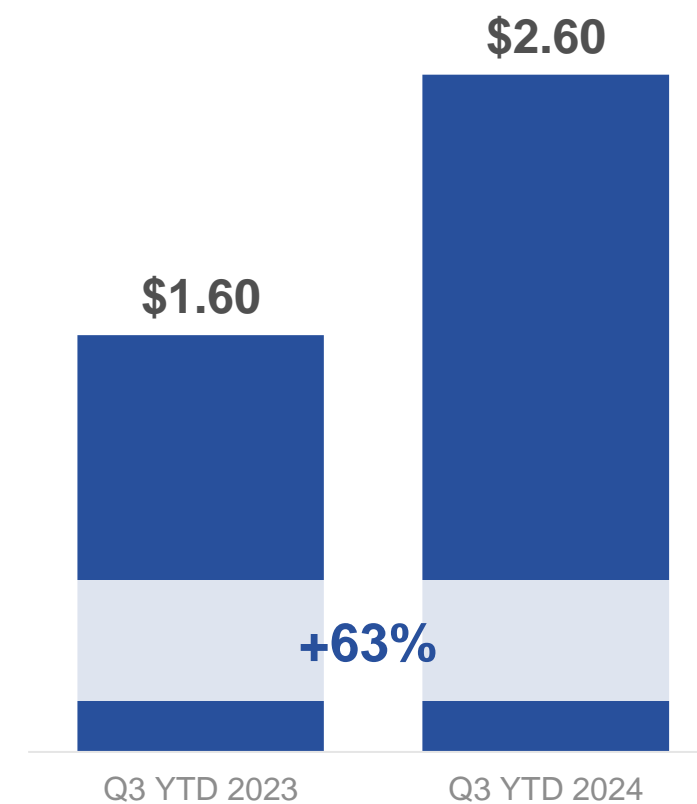
## Total Revenue Growth



## Non-GAAP Operating Margin %<sup>(1)</sup>



## Non-GAAP Diluted EPS<sup>(1)</sup>



(1) Refer to slide 4 and the appendix to this presentation for the definitions of Non-GAAP Operating Margin percentage and Non-GAAP Diluted EPS and reconciliations to the comparable information reported under U.S. GAAP.



# Updated Guidance for 2024

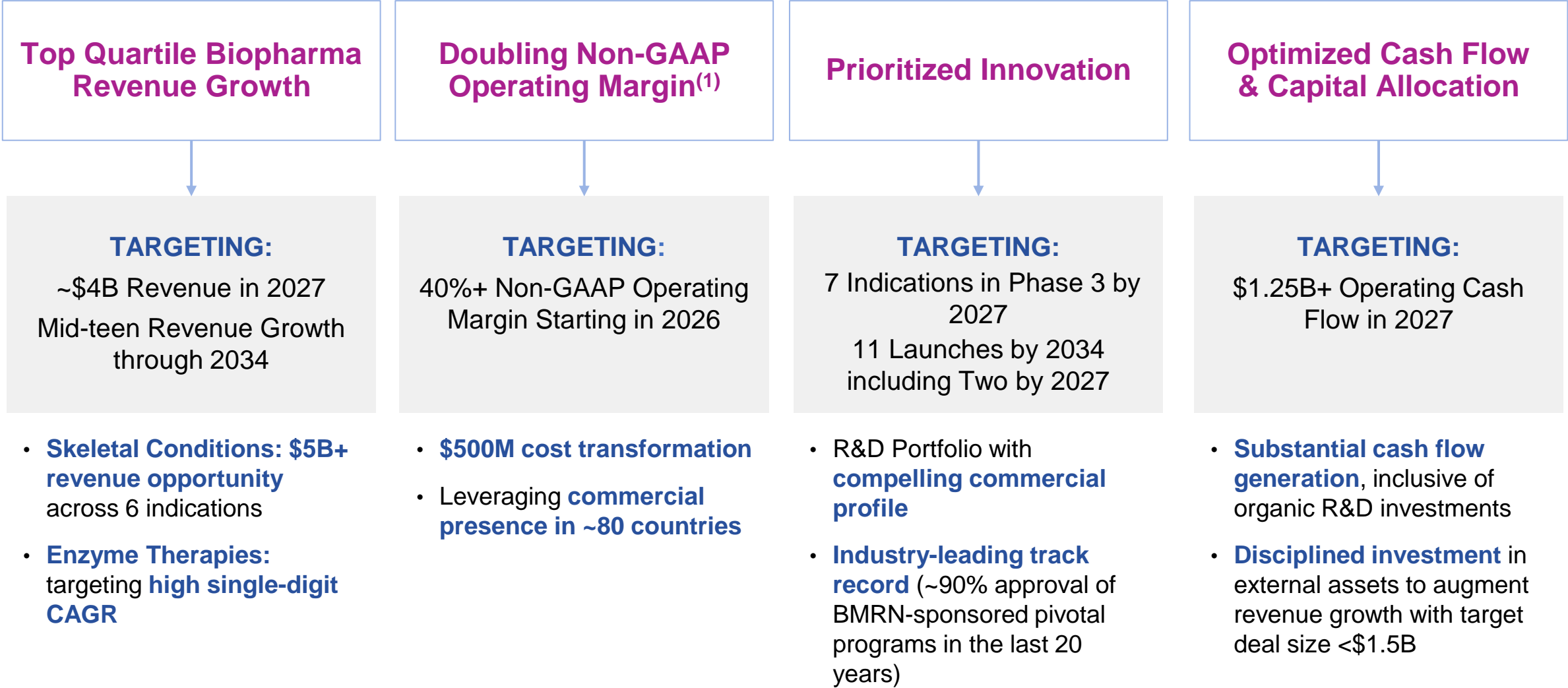
	Prior Guidance As of August 5, 2024	Updated Guidance <sup>(3)</sup> As of October 29, 2024
<i>(In millions, except per share and % data)</i>		
<b>Total Revenues</b>	\$2,750 - \$2,825	\$2,790 - \$2,825
<b>Non-GAAP Operating Margin %<sup>(1)</sup></b>	26% - 27%	26.5% - 27.5%
<b>Non-GAAP Diluted EPS<sup>(1)(2)</sup></b>	\$3.10 - \$3.25	\$3.25 - \$3.35

(1) Refer to slide 4 and the appendix to this presentation for the definitions of Non-GAAP Operating Margin percentage and Non-GAAP Diluted EPS.

(2) Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding.

(3) BioMarin does not provide guidance for GAAP reported financial measures (other than revenue) or a reconciliation of forward-looking Non-GAAP financial measures to the most directly comparable GAAP reported financial measures because the company is unable to predict with reasonable certainty the financial impact of changes resulting from its strategic portfolio and business operating model reviews; potential future asset impairments; gains and losses on investments; and other unusual gains and losses without unreasonable effort. These items are uncertain, depend on various factors, and could have a material impact on GAAP reported results for the guidance period. As such, any reconciliations provided would imply a degree of precision that could be confusing or misleading to investors.

# BioMarin Reaffirms Long-term Outlook Provided September 4, 2024



(1) Refer to slide 4 of this presentation for the definition of Non-GAAP Operating Margin percentage. BioMarin does not provide guidance for GAAP reported financial measures (other than revenue) or a reconciliation of forward-looking Non-GAAP financial measures to the most directly comparable GAAP reported financial measures because the company is unable to predict with reasonable certainty the financial impact of changes resulting from its strategic portfolio and business operating model reviews; potential future asset impairments; gains and losses on investments; and other unusual gains and losses without unreasonable effort. These items are uncertain, depend on various factors, and could have a material impact on GAAP reported results for the guidance period. As such, any reconciliations provided would imply a degree of precision that could be confusing or misleading to investors.

## Appendix

### **Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information**

# Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information<sup>(1)</sup>

	Three Months Ended September 30,				Nine Months Ended September 30,			
	Percent of GAAP Total Revenue		Percent of GAAP Total Revenue		Percent of GAAP Total Revenue		Percent of GAAP Total Revenue	
	2024	2023	2024	2023	2024	2023	2024	2023
<b>GAAP Income from Operations</b>	<b>\$ 114</b>	<b>15.3 %</b>	<b>\$ 31</b>	<b>5.3 %</b>	<b>\$ 323</b>	<b>15.3 %</b>	<b>\$ 158</b>	<b>8.9 %</b>
Adjustments								
Stock-based compensation expense	43	5.7	48	8.3	150	7.2	152	8.6
Amortization of intangible assets	5	0.7	16	2.8	34	1.6	47	2.7
Gain on sale of nonfinancial assets <sup>(2)</sup>	—	—	—	—	(10)	(0.5)	—	—
Severance and restructuring costs <sup>(3)</sup>	45	6.0	(1)	(0.2)	87	4.1	(1)	(0.1)
<b>Non-GAAP Income from Operations</b>	<b>\$ 207</b>	<b>27.7 %</b>	<b>\$ 94</b>	<b>16.2 %</b>	<b>\$ 583</b>	<b>27.7 %</b>	<b>\$ 357</b>	<b>20.1 %</b>

(1) Certain amounts may not sum or recalculate due to rounding.

(2) Represents a payment triggered by a third party's attainment of a regulatory approval milestone related to previously sold intangible assets.

(3) These amounts were included in SG&A and represent severance and restructuring costs related to the company's 2024 portfolio strategy review and the associated organizational redesign efforts announced in the second and third quarters of 2024. These amounts also include impairments of certain right-of-use and fixed assets.

# Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information<sup>(1)</sup>

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
<b>GAAP Diluted EPS</b>	<b>\$ 0.55</b>	<b>\$ 0.21</b>	<b>\$ 1.56</b>	<b>\$ 0.77</b>
Adjustments				
Stock-based compensation expense	0.22	0.24	0.76	0.76
Amortization of intangible assets	0.03	0.08	0.17	0.24
Gain on sale of nonfinancial assets <sup>(2)</sup>	—	—	(0.05)	—
Severance and restructuring costs <sup>(3)</sup>	0.22	—	0.44	—
Loss on investments <sup>(4)</sup>	—	—	0.03	0.06
Income tax effect of adjustments	(0.11)	(0.07)	(0.31)	(0.23)
<b>Non-GAAP Diluted EPS</b>	<b>\$ 0.91</b>	<b>\$ 0.46</b>	<b>\$ 2.60</b>	<b>\$ 1.60</b>

(1) Certain amounts may not sum or recalculate due to rounding.

(2) Represents a payment triggered by a third party's attainment of a regulatory approval milestone related to previously sold intangible assets.

(3) These amounts were included in SG&A and represent severance and restructuring costs related to the company's 2024 portfolio strategy review and the associated organizational redesign efforts announced in the second and third quarters of 2024. These amounts also include impairments of certain right-of-use and fixed assets.

(4) Represents a downward adjustment to non-marketable equity securities recorded in Other income (expense), net.

# Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information<sup>(1)</sup>

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
<b>GAAP Weighted-Average Diluted Shares Outstanding</b>	<b>197.1</b>	<b>191.2</b>	<b>196.7</b>	<b>195.0</b>
Adjustments				
Common stock issuable under the company's convertible debt <sup>(1)</sup>	—	8.4	—	4.4
<b>Non-GAAP Weighted-Average Diluted Shares Outstanding</b>	<b>197.1</b>	<b>199.6</b>	<b>196.7</b>	<b>199.4</b>

(1) Common stock issuable under the company's convertible debt was excluded from the computation of GAAP Weighted-Average Diluted Shares Outstanding when they were anti-dilutive. If converted, for the prior year comparative period, the company would have issued approximately 4.4 million shares under the convertible notes due in 2027 and 4 million shares under the convertible notes that matured on August 1, 2024.