

# Third Quarter 2023 Earnings

*November 1, 2023*



**BIOMARIN**<sup>®</sup>

*Transforming Lives Through  
Genetic Discovery*

# Table of Contents

**Business Overview & Highlights**

**5**

**Q3 '23 Key Financial Metrics**

**8**

**2023 Outlook**

**10**

**Non-GAAP Reconciliations**

**11**

# Forward-Looking Statements

This non-confidential presentation contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements regarding expectations of growth and profitability, expectations for results of operations and financial guidance for the full-year 2023 and BioMarin's clinical development and commercial prospects, including, without limitation, the risk of competition and long-lived revenue streams for BioMarin's enzyme-based therapy products, the markets for ROCTAVIAN and VOXZOGO, and the potential to leverage VOXZOGO in expanded age ranges and in conditions beyond achondroplasia, such as hypochondroplasia. 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 November 1, 2023 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, 2022 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, 2023. 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

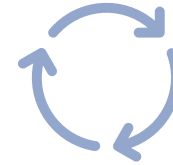
This presentation includes both GAAP information and Non-GAAP information. Non-GAAP Income is defined by the company as GAAP Net Income excluding amortization expense, stock-based compensation expense, contingent consideration expense, and, in certain periods, certain other specified items, as detailed below when applicable. The company also includes a Non-GAAP adjustment for the estimated tax impact of the reconciling items. For BioMarin's 2023 financial guidance, Non-GAAP Income is defined as GAAP Net Income excluding amortization of intangible assets, stock-based compensation expense, and certain other specified items that are typically highly variable, difficult to predict, and/or of a size that could have a substantial impact on the company's reported operations for a period, and Non-GAAP Diluted EPS is defined by the company as Non-GAAP Income divided by Non-GAAP diluted shares outstanding.

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.

# BioMarin is a Best-In-Class Genetic Disease Company at an Inflection Point of Transformative Growth

## Solid, Profitable, “IRA-proof” & Growing Base Business

- Anchored by durable enzyme-based therapies producing nearly \$2B of revenue and positive operating cash flows. Steady revenue growth across the portfolio
- High barriers to entry results in lower risk of competition and long-lived revenue streams



## New Large-market Launches Are Significant Near-Term Growth Drivers

- VOXZOGO® is the first and only approved treatment for Achondroplasia – tracking to blockbuster status
- ROCTAVIAN™, approved in the U.S. and Europe, is a safe and efficacious one-time treatment alternative to chronic therapy for eligible adults with severe hemophilia A
- Profit margin expansion expected as new product revenue growth leverages world class infrastructure



## Best-In-Class Innovation Capabilities

- Industry-leading R&D teams produced suite of eight internally-developed genetic-based commercial therapies
- Fully-integrated and scaled biopharma capabilities: Discovery, Clinical, Regulatory and world-class Manufacturing
- Leveraging decades of genetic expertise in current age of rapid genetic disease discovery
- Exceptional development success rate applied to deep early-stage pipeline with most assets in company history



# Executing On Our Strategy

Delivering top and bottom-line growth while fueling a sustainable pipeline of innovative products



# Third Quarter 2023 Key Business Highlights



## **Strong VOXZOGO® Demand Drove Profitability and Gross Margin Improvement**

\$123.1 million in VOXZOGO revenues drove total BioMarin GAAP net income of \$40.4 million and gross margin of 78.5% in the third quarter



## **Total Revenues of \$581.3 million in the Third Quarter 2023 Increased 15% Year-Over-Year**

Tracking to double-digit revenue growth and significant operating leverage, with more than 30% growth expected in bottom-line profitability in the full year 2023; For full-year 2024, BioMarin expects revenues to approach \$3 billion



## **VOXZOGO Approved for Children without Age Restrictions in the U.S. and for Children Aged 4 Months and Older in Europe**

Expanded age range unlocks an additional ~1,000 potential patients in the U.S. and Europe



## **New Phase 3 Study with VOXZOGO in Second Indication (Hypochondroplasia) Progressing**

Enrollment in the baseline, run-in arm of the study expected to begin in the fourth quarter of 2023



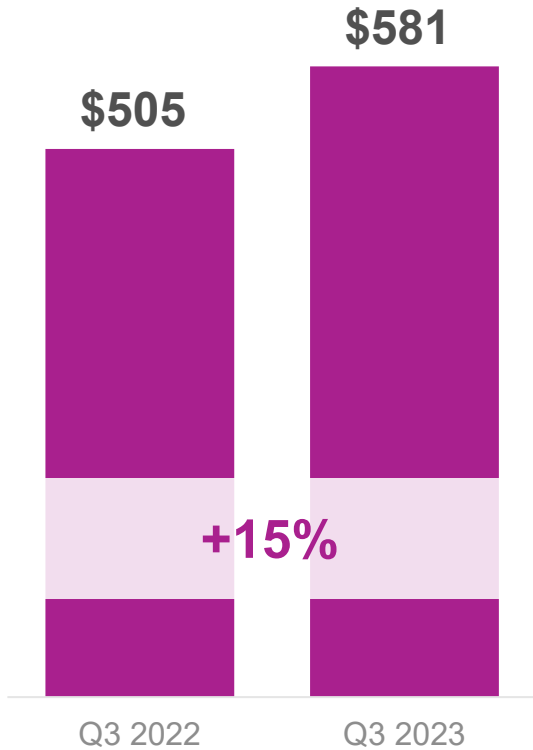
## **Two Patients Treated in Germany with ROCTAVIAN™; Final German & Italian Prices Agreed, Ratification Expected by Year End; Over 100 Treatment Eligibility Tests Completed Globally**

More than 205M U.S. with payer coverage at end of the third quarter; Multiple U.S. Hemophilia Treatment Centers ready to treat; Over 60 people in Germany with completed CDx tests known to be eligible for treatment with ROCTAVIAN

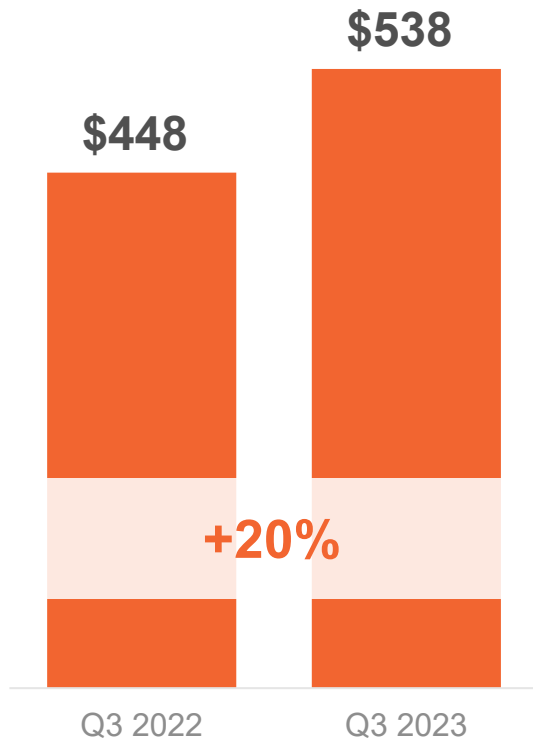
# Third Quarter 2023 Key Financial Metrics

(In millions, except percentage data)

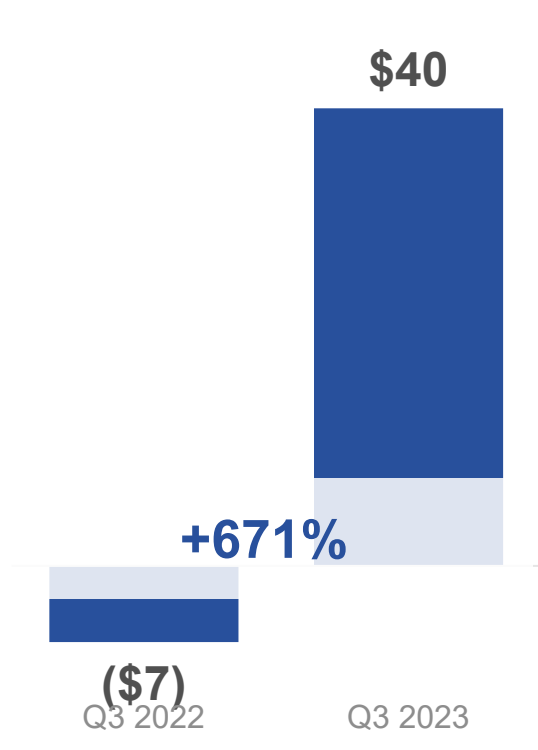
## Total Reported Revenue Growth



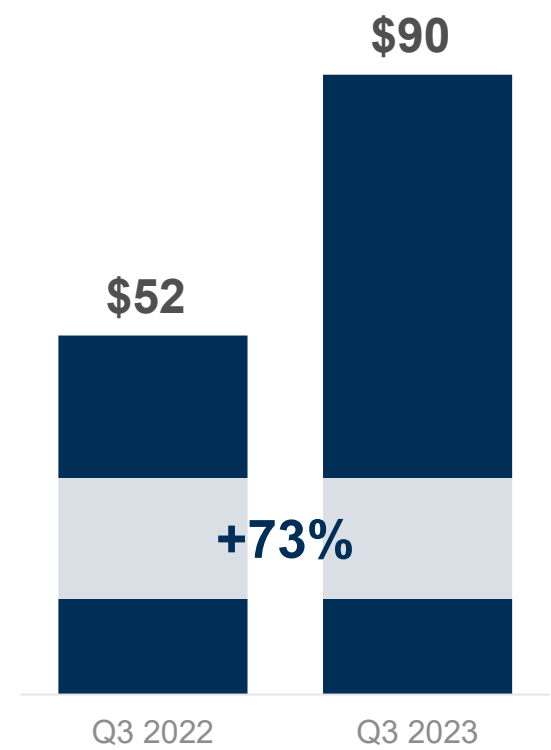
## Total Revenue Growth<sup>1</sup> Excluding KUVAN<sup>®</sup>



## GAAP Net Income



## Non-GAAP Net Income<sup>2</sup>



<sup>1</sup> Includes total revenues excluding revenues from KUVAN of \$42.9 million and \$57.0 million for the third quarter of 2023 and 2022, respectively.

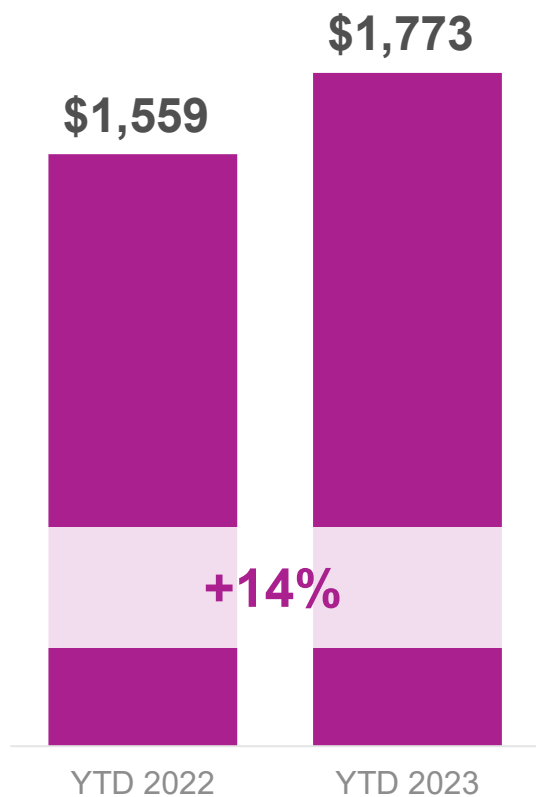
<sup>2</sup> Non-GAAP Income is defined by the company as reported GAAP Net Income, excluding amortization expense, stock-based compensation expense, contingent consideration, and, in certain periods, certain other specified items. The company also includes a Non-GAAP adjustment for the estimated income tax impact of reconciling items. Refer to Non-GAAP Information on slide 11 of this presentation for reconciliations to the comparable information reported under U.S. GAAP.



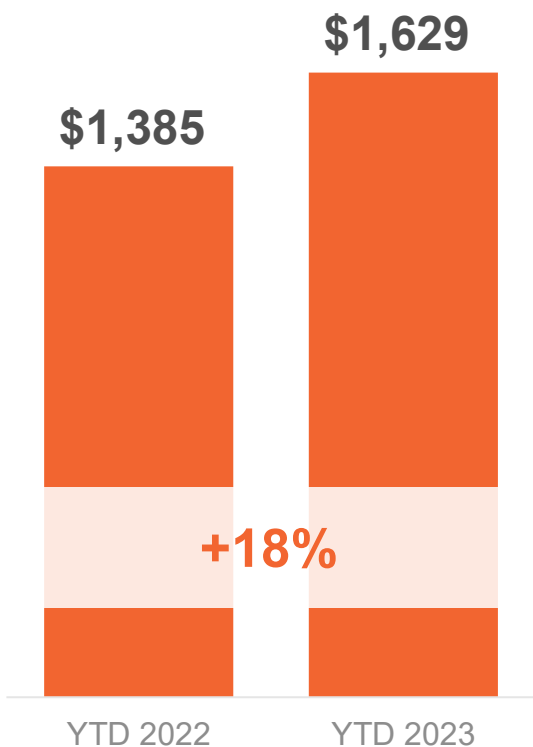
# Third Quarter Year-to-Date 2023 Key Financial Metrics

(In millions, except percentage data)

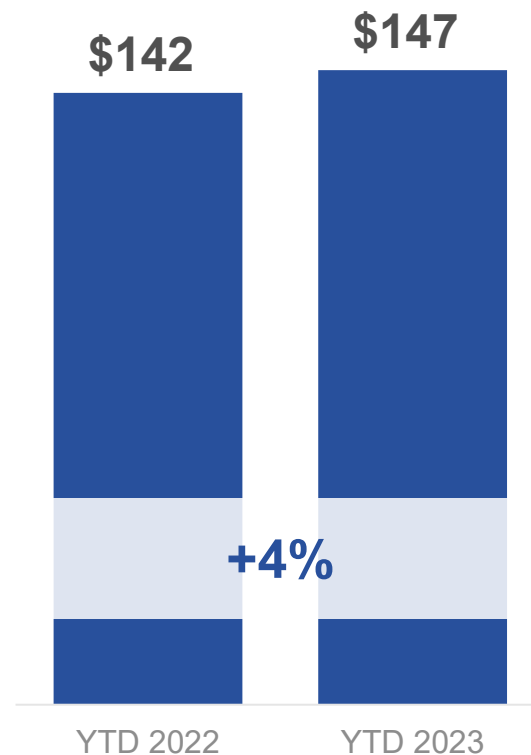
## Total Reported Revenue Growth



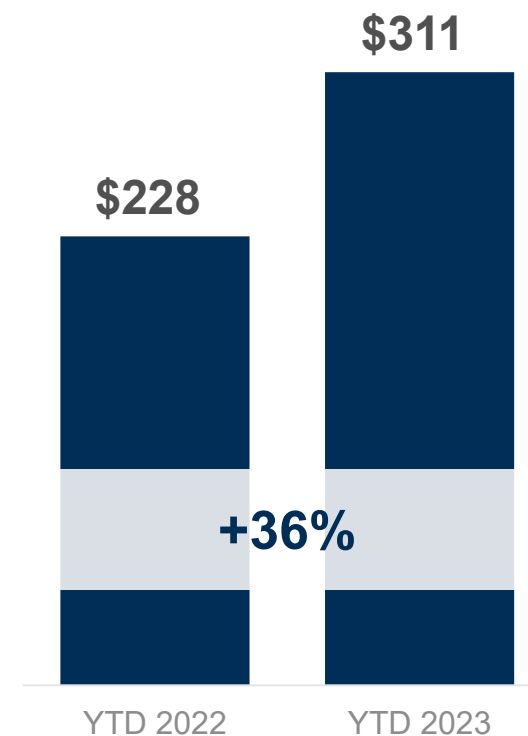
## Total Revenue Growth<sup>1</sup> Excluding KUVAN®



## GAAP Net Income<sup>2</sup>



## Non-GAAP Net Income<sup>3</sup>



<sup>1</sup> Includes total revenues excluding revenues from KUVAN of \$144.0 million and \$174.0 million for year-to-date periods 2023 and 2022, respectively.

<sup>2</sup> GAAP Net income in year to date in 2022 period included a \$89.0 million net gain, net of taxes, related to the sale of the Rare Pediatric Disease Priority Review Voucher (PRV) the company received from the U.S. Food and Drug Administration (FDA) in connection with U.S. approval of VOXZOGO.

<sup>3</sup> Non-GAAP Income is defined by the company as reported GAAP Net Income, excluding amortization expense, stock-based compensation expense, contingent consideration, and, in certain periods, certain other specified items. The company also includes a Non-GAAP adjustment for the estimated income tax impact of reconciling items. Refer to Non-GAAP Information on slide 12 of this presentation for reconciliations to the comparable information reported under U.S. GAAP.

# Full Year 2023 Guidance

(In millions, except per share and percentage data)

	<b>Prior Guidance</b> As of July 31, 2023	<b>Updated Guidance</b> As of November 1, 2023
Enzyme Products Revenue <sup>1</sup>	\$1,700 - \$1,850	\$1,700 - \$1,775
ROCTAVIAN Revenue	\$50 - \$150	less than \$10M
VOXZOGO Revenue	\$400 - \$440	\$435 - \$455
<b>Total Revenues</b>	<b>\$2,375 - \$2,500</b>	<b>\$2,390 - \$2,470</b>
Gross Margin %	77.5% - 79%	77.8% - 79%
R&D % of Revenue	30% - 32%	Unchanged
SG&A % of Revenue	35.5% - 37.5%	36% - 38%
<b>GAAP Net Income</b>	<b>\$165 - \$215</b>	<b>\$170 - \$210</b>
<b>GAAP Diluted EPS</b>	<b>\$0.83 - \$1.08</b>	<b>\$0.85 - \$1.05</b>
<b>Non-GAAP Net Income</b>	<b>\$370 - \$420</b>	<b>\$380 - \$410</b>
<b>Non-GAAP Diluted EPS<sup>2</sup></b>	<b>\$1.85 - \$2.10</b>	<b>\$1.90 - \$2.05</b>

<sup>1</sup> Enzyme Products include ALDURAZYME®, VIMIZIM®, NAGLAZYME®, BRINEURA®, and PALYNZIQ®.

<sup>2</sup> 2023 GAAP and Non-GAAP Diluted EPS guidance assumes ~200 million shares.

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

(In millions)

	Q3 2023	Q3 2022
GAAP Reported Net Income	\$ 40.4	\$ (6.7)
Adjustments		
Stock-based compensation expense - COS	4.0	4.4
Stock-based compensation expense - R&D	14.5	17.1
Stock-based compensation expense - SG&A	29.9	33.2
Amortization of intangible assets	15.7	15.9
Contingent consideration	—	0.9
Severance and employee termination benefits <sup>2</sup>	(0.4)	4.8
Income tax effect of adjustments	(14.6)	(17.6)
Non-GAAP Net Income	\$ 89.5	\$ 52.0

<sup>1</sup> Certain amounts may not sum or recalculate due to rounding.

<sup>2</sup> Represents change in estimates to severance and employee termination benefit charges in SG&A related to the company's organizational redesign announced in October 2022. Related to this October 2022 reorganization plan, the company recognized \$23.0 million of expense related to severance and employee termination benefits in 2022, the majority of which was recognized in the fourth quarter.

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

(In millions)

	YTD 2023	YTD 2022
GAAP Reported Net Income	\$ 147.3	\$ 141.8
Adjustments		
Stock-based compensation expense - COS	13.1	13.4
Stock-based compensation expense - R&D	49.4	47.9
Stock-based compensation expense - SG&A	89.8	88.2
Amortization of intangible assets	47.0	47.1
Contingent consideration	—	3.8
Gain on sale of non-financial assets <sup>2</sup>	—	(108.0)
Severance and employee termination benefits <sup>3</sup>	(0.5)	4.8
Loss on investment in equity securities <sup>4</sup>	12.6	—
Income tax effect of adjustments	(48.2)	(11.4)
Non-GAAP Net Income	\$ 310.5	\$ 227.6

<sup>1</sup> Certain amounts may not sum or recalculate due to rounding.

<sup>2</sup> Represents the net gain in the first quarter of 2022 on the sale to a third party of the PRV the company received from the FDA in connection with the U.S. approval of VOXZOGO.

<sup>3</sup> Represents change in estimates to severance and employee termination benefit charges in SG&A related to the company's organizational redesign announced in October 2022. Related to this October 2022 reorganization plan, the company recognized \$23.0 million of expense related to severance and employee termination benefits in 2022, the majority of which was recognized in the fourth quarter.

<sup>4</sup> Represents the impairment loss on investment in non-marketable equity securities recorded in Other income (expense), net in the first quarter of 2023.

# Reconciliation of GAAP Net Income to Non-GAAP Income and Dilutive EPS Guidance<sup>1</sup>


(In millions, except per share data)

	2023 Guidance <sup>1,2</sup>		
	Net Income	Diluted Shares	Diluted EPS
GAAP Net Income and Diluted EPS	<b>\$170 to \$210</b>	<b>200</b>	<b>\$0.85 to \$1.05</b>
Adjustments			
Stock-based compensation expense	60		0.30
Amortization of intangible assets	200		1.00
Severance and employee termination benefits	(0.5)		(0.00)
Loss on investment in equity securities	12.6		0.06
Income tax effect of adjustments <sup>3</sup>	(67)		(0.34)
Non-GAAP Income and Diluted EPS	<b><u>\$380 to \$410</u></b>	<b>200</b>	<b><u>\$1.90 to \$2.05</u></b>

1 The adjustments/reconciling items included in this table are presented to facilitate the reconciliation of Non-GAAP Income and Non-GAAP Diluted EPS to their closest GAAP financial metrics, GAAP Net Income and GAAP Diluted EPS. The specific amounts included in each reconciling line item above represent approximations of the underlying adjustments from GAAP Net Income to Non-GAAP Income and from GAAP Diluted EPS to Non-GAAP Diluted EPS. Actual 2023 results for each reconciling line item may be different, in some cases materially, than the amounts listed above as a result of uncertainty regarding, and the potential variability of, those items.

2 Amounts will sum when using midpoint of guidance ranges.

3 Income tax adjustments represent the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable statutory income tax rate.



# Third Quarter 2023 Earnings

*November 1, 2023*



**B:OMARIN<sup>®</sup>**

*Transforming Lives Through  
Genetic Discovery*