First Quarter 2025 Earnings

May 1, 2025

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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, Non-GAAP Diluted EPS and Operating Cash Flow for, in certain instances, the full-year 2025 and future periods, as well as profitability growth in 2025, and the underlying drivers of those results, such as the revenue opportunity represented by treatments for Skeletal Conditions, namely VOXZOGO, the expected demand and continued growth of BioMarin's Enzyme Therapies portfolio, including PALYNZIQ, and the expectation regarding the full realization of the benefits of BioMarin's cost transformation program; plans regarding BioMarin's revamped corporate strategy and operating model in 2025 and beyond, including expected growth in the Skeletal Conditions business unit and execution of BioMarin's business development strategy, 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) expected advancements of pipeline candidates, including BMN 333, BMN 349 and BMN 351, the anticipated initial data read-out for BMN 351 in the second half of 2025, the assumptions and expectations regarding predicted time course of dystrophin expression and anticipated dystrophin level at 25-week data cut with respect to BMN 351, and the expected data and data presentation for BMN 333 in the first half of 2026 and plans to initiate a registration-enabling study for BMN 333 in 2026, as well as plans to seek similar agreements with additional global regulators in the coming months; (ii) plans to submit applications to expand PALYNZIQ age eligibility for the treatment of adolescents with phenylketonuria between the ages of 12 and 17 in the U.S. and Europe in the second half of 2025; (iii) expected topline data from the pivotal study in hypochondroplasia in 2026 and launch in 2027; (iv) the expectations regarding global expansion of VOXZOGO for achondroplasia and expected increase in the rate of U.S. expansion in the second half of 2025; (v) plans to advance five new VOXZOGO indications with BioMarin's CANOPY clinical program; and (vi) plans to reach greater number of patients around the world across BioMarin's Enzyme Therapies; the expected benefits and availability of BioMarin's commercial products and product candidates; and potential growth opportunities and trends. 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 on May 1, 2025, 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, 2024 as such factors may be updated by any subsequent reports. 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.

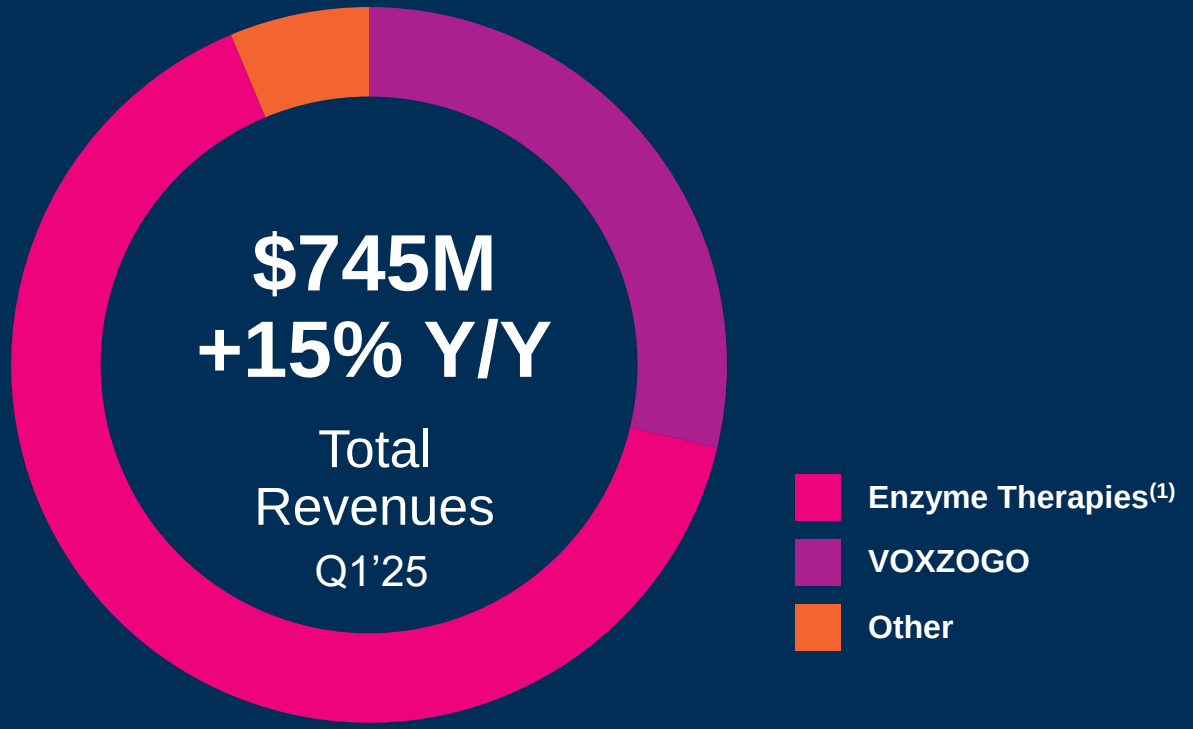
BioMarin regularly uses both GAAP and Non-GAAP results and expectations internally to assess its financial operating performance and evaluate key business decisions related to its principal business activities: the discovery, development, manufacture, marketing and sale of innovative biologic therapies. 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 financial measures 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 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.

With respect to historical Non-GAAP adjusted financial information, see the appendix beginning on slide 10 for the reconciliations to the comparable information reported under U.S. GAAP.

In Q1’25, BioMarin delivered **15% revenue growth** and **significant profitability** expansion, driven by execution across the business; **full year guidance reaffirmed**



First Quarter 2025 Results Y/Y	VOXZOGO Revenues	Enzyme Therapies(1) Revenues	GAAP Operating Margin	Non-GAAP Operating Margin(2)	GAAP Diluted Earnings per Share (EPS)	Non-GAAP Diluted EPS(2)
	$214M **+40%**	**$484M** **+8%**	**30.0%** **+16.4 ppts**	**35.7%** **+11.9 ppts**	**$0.95** **+107%**	**$1.13** **+59%**

All growth rates are compared to first quarter 2024
(1) Enzyme Therapies include ALDURAZYME, BRINEURA, NAGLAZYME, PALYNZIQ, and VIMIZIM
(2) Refer to slide 4 for more detail on Non-GAAP financial measures

Strong First Quarter 2025 Performance Across the Company

INNOVATION

- **Pivotal study with VOXZOGO in hypochondroplasia fully enrolled** in April 2025; **potential launch in 2027**
- **FDA aligned on development plan for BMN 333; plan to initiate registration-enabling study in 2026**
- **Initial data for BMN 351** for DMD expected to be presented at a scientific congress in **2H'25**
- **Positive pivotal topline results** for PALYNZIQ in adolescents ages 12-17 shared in April, supporting planned submissions to regulatory authorities in the U.S. and EU in **2H'25**

GROWTH

- **VOXZOGO** Q1 revenues increased **40%** Y/Y, driven by new patient additions across the globe
- As of Q1, children across **49** countries had access to VOXZOGO treatment; BioMarin on track to expand VOXZOGO access to more than **60** countries by 2027
- **Enzyme Therapies** Q1 revenues grew **8%** Y/Y, led by **PALYNZIQ's 22% growth** driven by strong demand

VALUE COMMITMENT

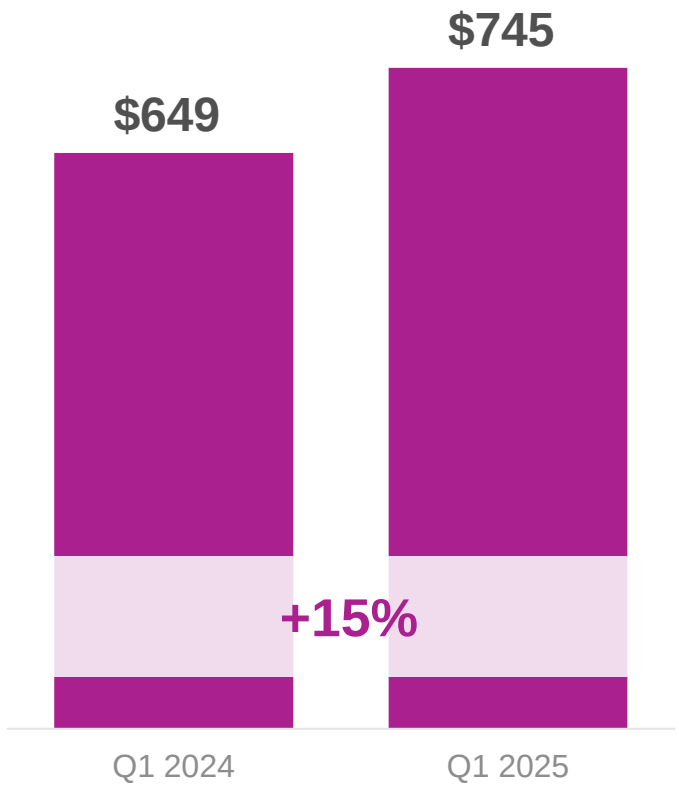
- Strong commercial performance resulted in **first quarter revenues of $745M**, **15% growth Y/Y**
- Revenue growth and focused operational execution drove Q1 Non-GAAP Operating Margin % of **35.7%** (+11.9 ppts Y/Y) and **operating cash flows** of **$174M** (+271% Y/Y), supporting **reinvestment into innovation**
- **FY'25 guidance** reaffirmed[(1)] for Total Revenues, Non-GAAP Operating Margin %[(2)], and Non-GAAP Diluted EPS[(2),(3)]

[(1)]Reaffirmed guidance reflects the impact of tariffs that have already been enacted but does not reflect the impact of potential future pharmaceutical tariffs; [(2)] Refer to slide 4 for more detail on Non-GAAP financial measures; [(3)]Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding.

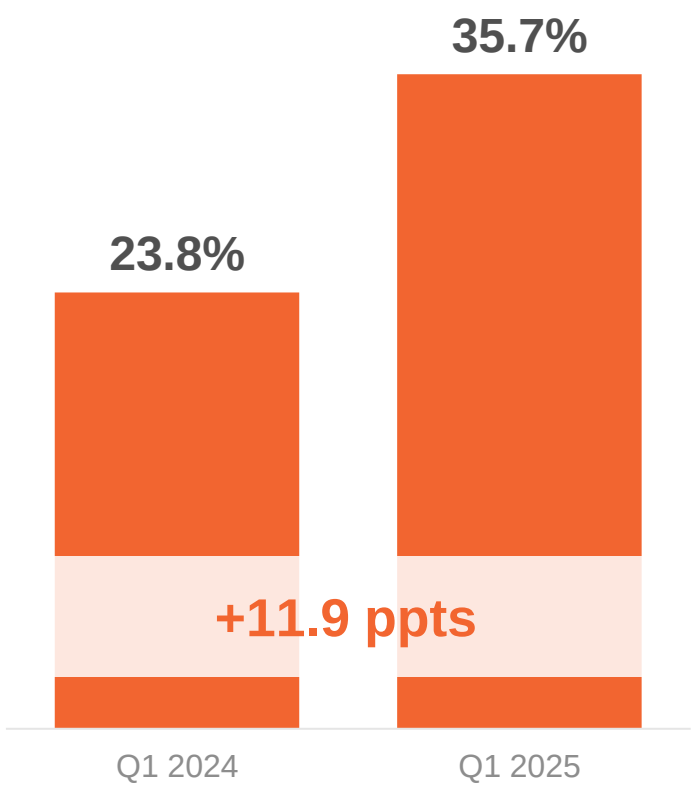
First Quarter 2025 Key Financial Metrics

(In millions, except share and percentage data)

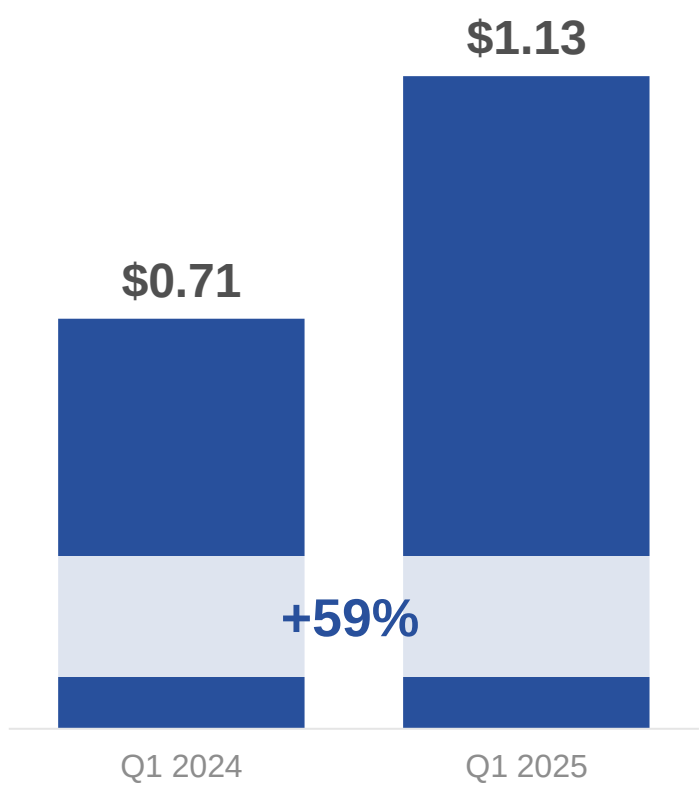
Total Revenue Growth



Non-GAAP Operating Margin %(1)



Non-GAAP Diluted EPS(1)



(1) Refer to slide 4 for more detail on Non-GAAP financial measures.

Guidance Reaffirmed for Full-year 2025

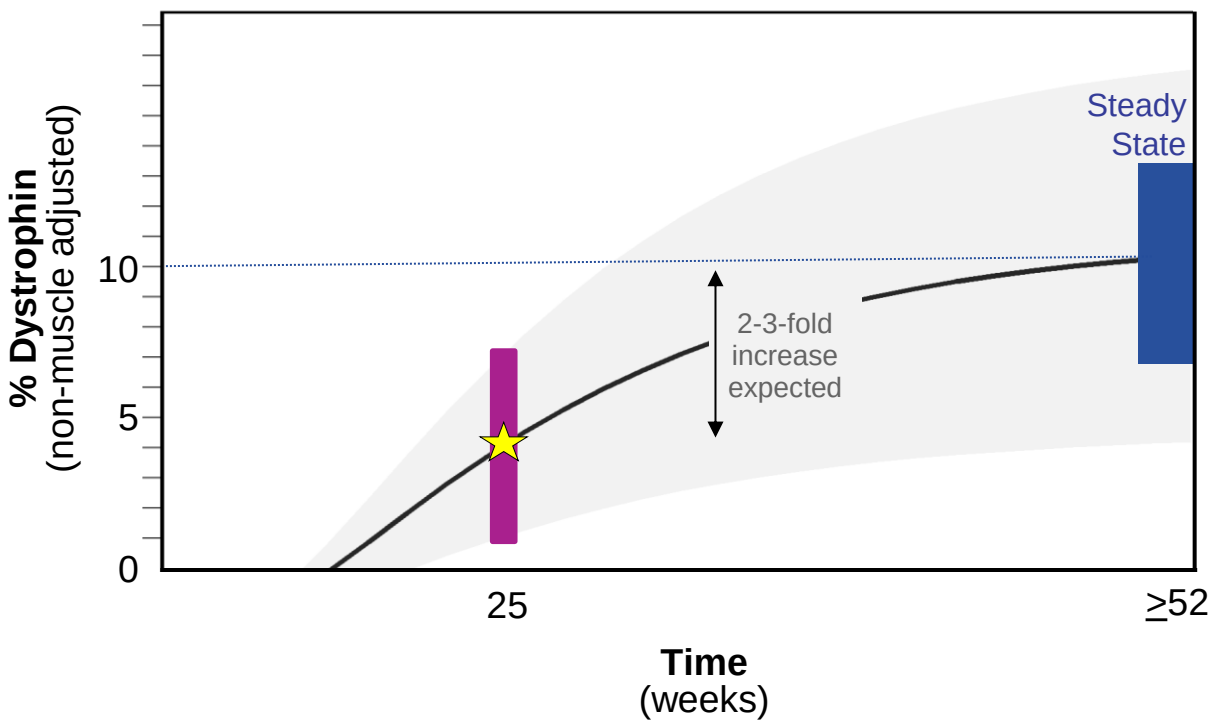
(In millions, except per share and % data)	2024 Actuals	2025 Guidance Issued February 19, 2025
Total Revenues	$2,854	$3,100 to $3,200
Non-GAAP Operating Margin %(1)	28.6%	32% to 33%
Non-GAAP Diluted EPS(1)(2)	$3.52	$4.20 to $4.40

Reaffirmed guidance reflects the impact of tariffs that have already been enacted but does not reflect the impact of potential future pharmaceutical tariffs

(1) Refer to slide 4 for more detail on Non-GAAP financial measures.

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

BMN 351: Predicted Time Course of Dystrophin Expression



Modeling assumptions (e.g. drug, exon skip product, and dystrophin half-lives) based on data from Del52/mdx mice, NHPs, and prior human experience in a phosphorothioate oligonucleotides study for DMD

- Steady state when BMN 351 and dystrophin accumulation at capacity in muscle tissues
- **2-3 fold increase in dystrophin expected between week 25 and steady state**
- Following Cohort 1, 25-week data readout, model and assumptions will be updated based on week 13 and week 25 BMN 351 clinical data
- Optimal doses for further clinical evaluation will be identified based on risk benefit assessment

Appendix

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information(1)

	Three Months Ended March 31,			
	2025	Percent of GAAP Total Revenue	2024	Percent of GAAP Total Revenue
GAAP Income from Operations	**$ 224**	**30.0 %**	**$ 88**	**13.6 %**
Adjustments				
Stock-based compensation expense	37	5.0	58	9.0
Amortization of intangible assets	5	0.7	14	2.2
Gain on sale of nonfinancial assets (2)	—	—	(10)	(1.5)
Severance and restructuring costs (3)	—	—	3	0.5
Non-GAAP Income from Operations	$ 266	35.7 %	$ 154	23.8 %

(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 corporate initiatives and the associated organizational redesign efforts.

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information[1]

	Three Months Ended March 31,	
	2025	2024
GAAP Diluted EPS	**$ 0.95**	**$ 0.46**
Adjustments		
Stock-based compensation expense	0.19	0.29
Amortization of intangible assets	0.03	0.07
Gain on sale of nonfinancial assets [2]	—	(0.05)
Severance and restructuring costs [3]	—	0.02
Loss on investments [4]	0.02	—
Income tax effect of adjustments	(0.05)	(0.08)
Non-GAAP Diluted EPS [5]	$ 1.13	$ 0.71

(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 corporate initiatives and the associated organizational redesign efforts.
(4) Represents an impairment loss on non-marketable equity securities recorded in Other income (expense), net.
(5) Non-GAAP Weighted-Average Diluted Shares Outstanding were 196.5 million and 199.3 million shares for the three months ended March 31, 2025 and 2024, respectively, which were equal to the respective GAAP Weighted-Average Diluted Shares Outstanding in the periods presented.