



First Quarter 2024 Earnings

April 24, 2024



B:OMARIN[®]

*Transforming Lives Through
Genetic Discovery*

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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, and Non-GAAP Diluted EPS for the full-year 2024; BioMarin's new corporate strategy, including the timing of the completion and announcement; BioMarin's ability to accelerate the VOXZOGO opportunity; the anticipated benefits from its ongoing strategic review; the timing of orders for commercial products; 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 as well as idiopathic short stature and other genetic short stature pathway conditions; the commercialization of BioMarin's products, including (i) the anticipated start and growth of commercial sales of VOXZOGO in additional countries, and (ii) the commercialization of ROCTAVIAN for the treatment of severe hemophilia A in the U.S. and Europe (including Italy and Germany); the expected benefits and availability of BioMarin's product candidates; and potential growth opportunities and trends, including BioMarin's expectation that VOXZOGO product growth will continue to expand rapidly. 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 April 24, 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. 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 diluted shares outstanding. The company's presentation of percentage changes in total revenues at constant currency rates, which is computed using current period local currency sales at the prior period's foreign exchange rates, is also a Non-GAAP financial measure. This measure provides information about growth (or declines) in the company's total revenue as if foreign currency exchange rates had not changed between the prior period and the current period.

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.

First Quarter 2024 Key Business Highlights



Q1 Record Results Were Driven by Global Demand for VOXZOGO & Strong Contributions from Other Products

VOXZOGO revenues of \$153M, increased 74% year-over-year



Total Revenues of \$649M in Q1 2024 Increased 9%, 13% at Constant Currency⁽¹⁾, Year-over-Year

Non-GAAP Diluted EPS grew twice as fast as total revenues



Children Treated with VOXZOGO Increased 102% Year-over-year

More than 500 children began VOXZOGO treatment in Q1'24, and totaled over 3,100 by quarter end; expansion continued across key large markets and, in the U.S. specifically, was driven by demand from younger age groups



R&D Prioritization Resulted in Focused Pipeline with Three Assets to be Accelerated

BMN 333, BMN 349, and BMN 351 chosen to accelerate based on the highest probability of success and benefit for patients



FY'24 Total Revenue Guidance Reaffirmed, Non-GAAP Operating Margin % & Non-GAAP Diluted EPS Guidance Raised ⁽²⁾⁽³⁾

As a result of the discontinuation of some R&D programs, Non-GAAP operating margin % guidance increased to 24% - 25% and full-year Non-GAAP diluted EPS guidance increased to \$2.75 - \$2.95

(1) Refer to slide 4 and the appendix to this presentation for the definitions of constant currency and reconciliations to the comparable information reported under U.S. GAAP.

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

(3) Non-GAAP Diluted EPS guidance assumes approximately 200 million weighted-average diluted shares outstanding.

First Quarter Update on 2024 Strategic Priorities



ACCELERATE AND MAXIMIZE THE VOXZOGO OPPORTUNITY

- Number of children treated with VOXZOGO increased globally in the quarter, supported by new prescriptions in the U.S. for children under the age of 5 years
- Enrollment in the observational portion of the registrational study with VOXZOGO for hypochondroplasia continued; ISS & PC studies to begin enrollment in the 2H'24



ESTABLISH ROCTAVIAN OPPORTUNITY

- Reimbursement and market access challenges continue to impact patient access to ROCTAVIAN treatment
- In April, BioMarin treated the first patient in Italy with ROCTAVIAN



FOCUS R&D ON THE MOST PRODUCTIVE ASSETS

- BioMarin completed its strategic portfolio assessment of R&D programs
- Focusing on patient impact and commercial opportunity, three programs to be accelerated, BMN 333, BMN 349, and BMN 351



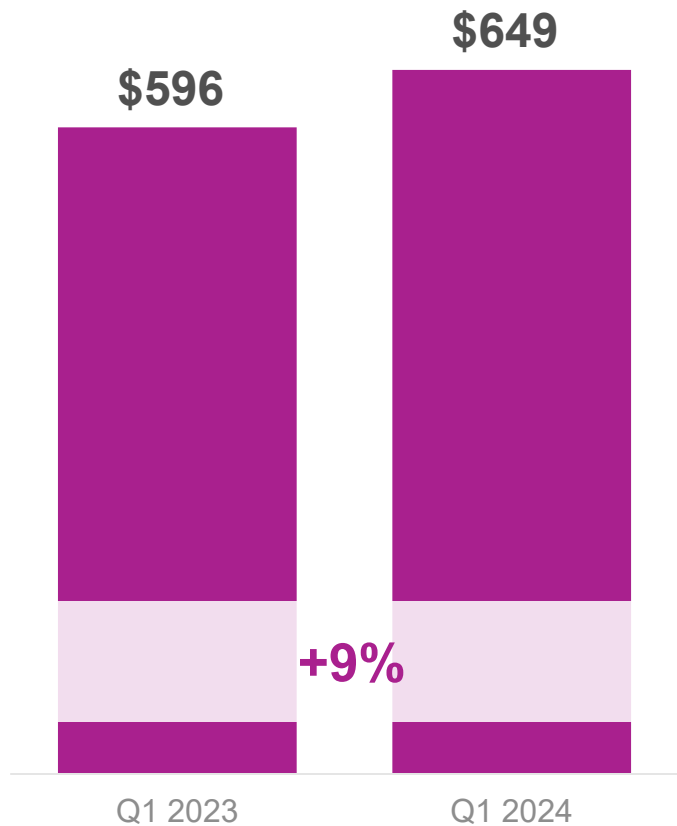
ACCELERATE EPS GROWTH AND EXPAND MARGINS

- Non-GAAP Operating Margin % expansion and Non-GAAP Diluted EPS grew twice as fast as total revenues
- Reaffirmed FY 2024 Total Revenues guidance and raised Non-GAAP Operating Margin % and Non-GAAP Diluted EPS guidance

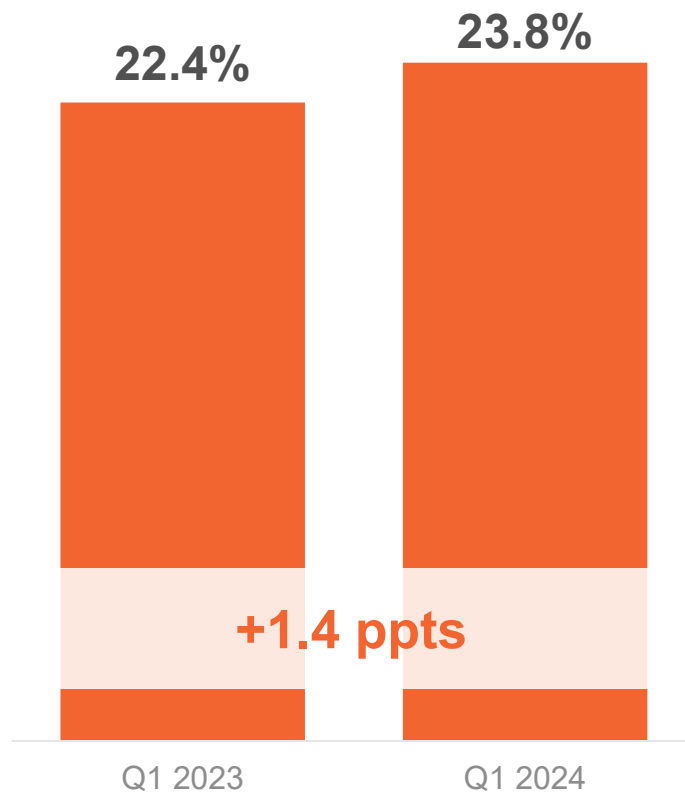
First Quarter 2024 Key Financial Metrics

(In millions, except share and percentage data)

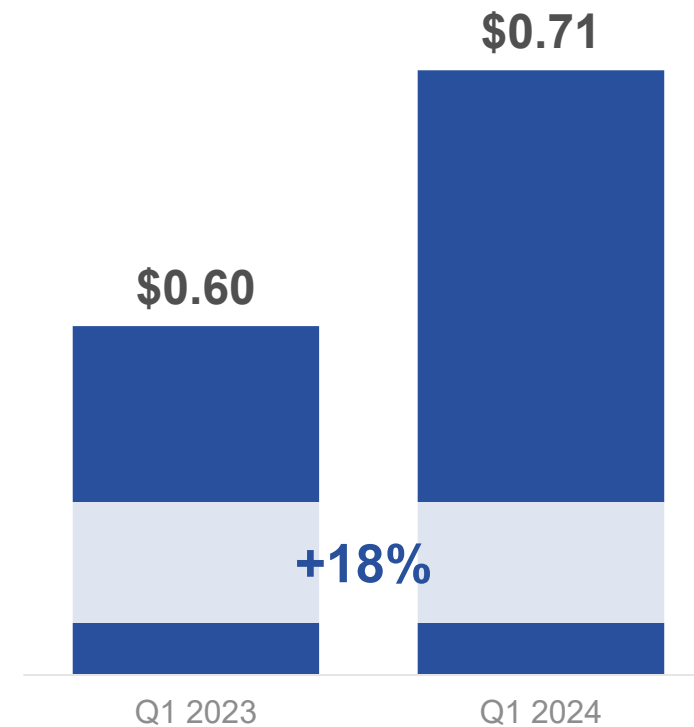
Total Revenue Growth



Non-GAAP Operating Margin %⁽¹⁾



Non-GAAP Diluted EPS⁽¹⁾



7 (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.

Evolution of our Communications Approach in 2024 and Beyond



Maturing Guided Line Items

- Total Revenues
- Non-GAAP Operating Margin %
- Non-GAAP Diluted EPS



More Consistent Cadence and Content of Updates

- Communicate business updates and objectives each quarter
- Focus on material developments and catalysts
- Scientific data presented at medical conferences and congresses



Investor Day 2024 (September 4, NYC)

- New corporate strategy, vision and long-term aspirations
- Strategic and Operating Review Committee outcomes
- Long-term financial targets

Updated Guidance for 2024

	Prior Guidance As of February 22, 2024	Updated Guidance As of April 24, 2024
<i>(In millions, except per share and % data)</i>		
Total Revenues	\$2,700 - \$2,800	No Change
Non-GAAP Operating Margin %⁽¹⁾	23% - 24%	24% - 25%
Non-GAAP Diluted EPS⁽¹⁾⁽²⁾	\$2.60 - \$2.80	\$2.75 to \$2.95



Appendix

Reconciliation of GAAP Reported to Selected Non-GAAP
Adjusted Information

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

	Three Months Ended March 31,	
	Dollar	Percentage
GAAP Change in Total Revenues	\$52.4	9%
Less: impact of foreign currency exchange rates on product sales denominated in currencies other than U.S. dollars	(22.7)	
Non-GAAP change in Total Revenues at Constant Currency	<u>\$75.1</u>	13%

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

	Three Months Ended March 31,			
	2024	Percent of GAAP Total Revenue	2023	Percent of GAAP Total Revenue
GAAP Income from Operations	\$ 88.5	13.6%	\$ 62.4	10.5%
Adjustments				
Stock-based compensation expense	58.2	9.0%	53.6	8.9%
Amortization of intangible assets	14.3	2.2%	15.7	2.6%
Gain on sale of nonfinancial assets ⁽²⁾	(10.0)	(1.5)%	-	-
Severance and restructuring costs ⁽³⁾	3.4	0.5%	2.1	0.4%
Total Non-GAAP adjustments	65.9	10.2%	71.4	11.9%
Non-GAAP Income from Operations	\$ 154.4	23.8%	\$ 133.8	22.4%

(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 company's organizational redesign announced in October 2022, respectively.

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

	Three Months Ended March 31,	
	2024	2023
GAAP Diluted EPS	\$ 0.46	\$ 0.27
Adjustments		
Stock-based compensation expense	0.29	0.27
Amortization of intangible assets	0.07	0.08
Gain on sale of nonfinancial assets ⁽²⁾	(0.05)	-
Severance and restructuring costs ⁽³⁾	0.02	0.01
Loss on investments ⁽⁴⁾	-	0.06
Income tax effect of adjustments	(0.08)	(0.09)
Non-GAAP Diluted EPS	\$ 0.71	\$ 0.60

(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 company's organizational redesign announced in October 2022, respectively.

(4) Represents an impairment loss on non-marketable equity securities recorded in Other income (expense), net.

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

	<u>Three Months Ended March 31,</u>	
	<u>2024</u>	<u>2023</u>
GAAP Weighted-Average Dilutive Shares Outstanding	199.3	194.4
Adjustments		
Common stock issuable under company's convertible debt ⁽¹⁾		4.4
Non-GAAP Weighted-Average Dilutive Shares Outstanding	<u>199.3</u>	<u>198.8</u>



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