First Quarter 2023 Earnings

April 26, 2023

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Transforming Lives Through Genetic Discovery

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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, (i) the risk of competition and long-lived revenue streams for BioMarin's enzyme-based therapy products, (ii) the markets for ROCTAVIAN and VOXZOGO and (iii) the duration and outcome of the FDA's review of BioMarin's BLA resubmission for ROCTAVIAN. 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 26, 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. 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, 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.

The reclassified 2022 and 2021 non-GAAP information calculated consistent with the 2023 Non-GAAP Income methodology is available at Biomarin.com. As of April 26, 2023, the workbook has been updated to conform to our current presentation.

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 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® as first treatment for Achondroplasia – BioMarin’s strongest global launch to date
- ROCTAVIAN™ poised to transform treatment for Severe Hemophilia A with First Gene Therapy to launch in a large, chronically managed population with unmet medical needs
- 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

First Quarter 2023 Key Business Highlights

Record Revenues in First Quarter, with 15% Year-Over-Year Growth

Tracking to double-digit revenue growth and significant operating leverage, driving approximately 30% growth in expected bottom-line profitability in full-year 2023

Strong Profitability and Gross Margin Improvement Driven by Robust VOXZOGO® Uptake

$88 million in VOXZOGO revenues drove Total BioMarin Net Income of $51 million and gross margin of 78.8% in the first quarter

VOXZOGO® Revenues More than Quadrupled versus First Quarter of 2022

Increased full-year 2023 VOXZOGO guidance by $50 million from the mid-point, representing 140% growth year-over-year

Outside the U.S., ROCTAVIAN™ Commercial Launch Progressing

Growing pipeline of people with severe Hemophilia A getting tested for ROCTAVIAN treatment eligibility

ROCTAVIAN™ U.S. 3-Year Phase 3 Results Submitted to FDA – Met all Efficacy Endpoints

U.S. Prescription Drug User Fee Act (PDUFA) target action date currently set for June 30, 2023

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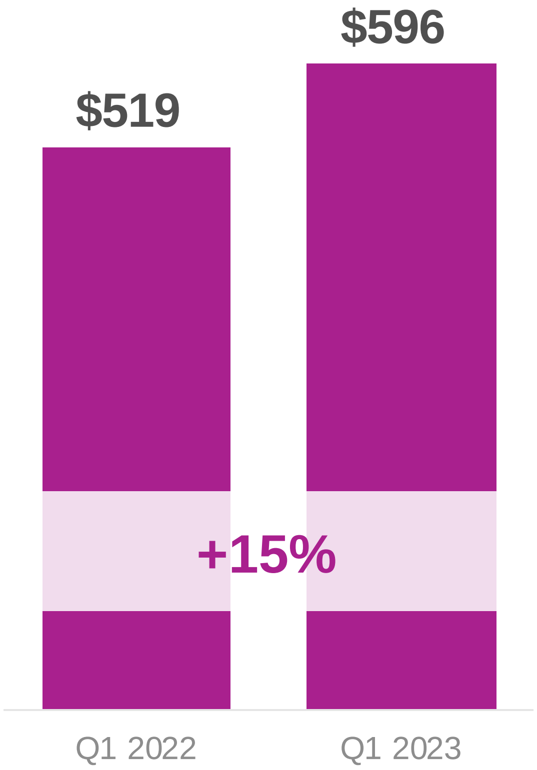
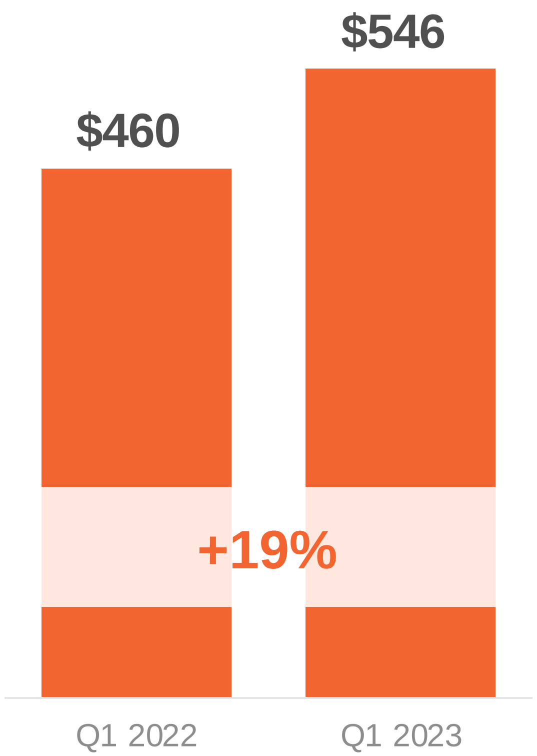
First Quarter 2023 Key Financial Metrics

(In millions, except percentage data)

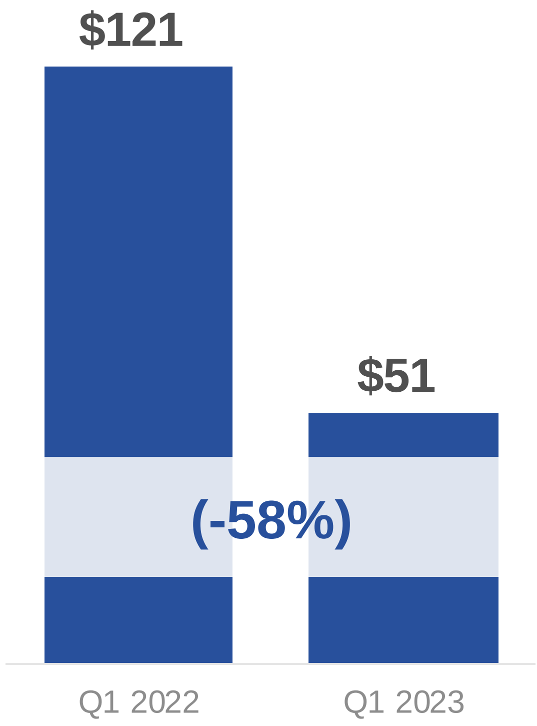
Reported Revenue Growth



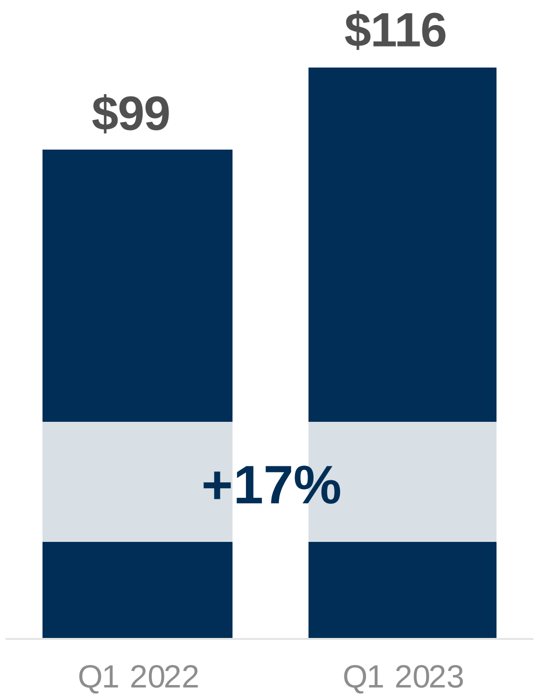
Revenue Growth[1] Excluding KUVAN®



GAAP Net Income[2]



Non-GAAP Net Income



1 Includes total revenues excluding revenues from KUVAN.
2 The first quarter of 2022 includes the net gain on the sale to a third party of the Rare Pediatric Disease Priority Review Voucher (PRV) the Company received from the FDA in connection with the U.S. approval of VOXZOGO.

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Full Year 2023 Guidance

(In millions, except per share and percentage data)	**Prior Guidance** **As of February 27, 2023**	**Current Guidance[1]** **As of April 26, 2023**
Enzyme Products[2]	\$1,700 - \$1,850	Unchanged
ROCTAVIAN	\$100 - \$200	\$50 - \$150
VOXZOGO	\$330 - \$380	\$380 - \$430
Total Revenues	**\$2,375 - \$2,500**	**Unchanged**
Gross Margin %	77.5% - 79.0%	**Unchanged**
R&D % of Revenue	30% - 32%	**Unchanged**
SG&A % of Revenue	36% - 38%	**Unchanged**
GAAP Net Income	**\$155 - \$205**	**Unchanged**
GAAP Diluted EPS	**\$0.78 - \$1.03**	**Unchanged**
Non-GAAP Net Income	**\$360 - \$410**	**Unchanged**
Non-GAAP Diluted EPS[3]	**\$1.80 - \$2.05**	**Unchanged**

1 The full-year 2023 ROCTAVIAN guidance range, provided above, represents global revenue estimates and assumes a U.S. approval in June 2023.
2 Enzyme Products include ALDURAZYME ®, VIMIZIM®, NAGLAZYME®, BRINEURA®, and PALYNZIQ®.
3 2023 GAAP and Non-GAAP Diluted EPS guidance assumes ~200 million shares.

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Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

(In millions)

	Q1 2023	Q1 2022
GAAP Reported Net Income	$ 51	$ 121
Adjustments		
Stock-based compensation expense - COS	4	4
Stock-based compensation expense - R&D	20	17
Stock-based compensation expense - SG&A	30	26
Amortization of intangible assets	16	16
Contingent consideration	-	2
Gain on sale of non-financial assets[1]	-	(108)
Severance and employee termination benefits[2]	2	-
Loss on investment in equity securities[3]	13	-
Income tax effect of adjustments	(19)	21
Non-GAAP Net Income	$ 116	$ 99

1 Represents the net gain on the sale in the first quarter of 2022 to a third party of the PRV the Company received from the FDA in connection with the U.S. approval of VOXZOGO.
2 Represents severance and employee termination benefit charge in SG&A related to the Company's organizational redesign announced in October 2022. The Company recognized $23.0 million of expense related to severance and employee termination benefits in 2022.
3 Represents the impairment loss on investment in non-marketable equity securities recorded in Other expense, net in the first quarter of 2023.

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Reconciliation of GAAP Net Income to Non-GAAP Income and Dilutive EPS Guidance

(In millions, except per share data)

	2023 Guidance[1,2]		
	Net Income	Diluted Shares	Diluted EPS
GAAP Net Income and Diluted EPS	$155 to $205	200	$0.78 to $1.03
Amortization of intangible assets	60		$0.30
Stock-based compensation expense	200		$1.00
Severance and employee termination benefits	2.1		$0.01
Loss on investment in equity securities	12.6		$0.06
Income tax effect of adjustments[3]	(70)		($0.35)
Non-GAAP Income and Diluted EPS	$360 to $410	200	$1.80 to $2.05

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 Certain amounts may not sum or recalculate due to rounding.

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.

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