

# Fourth Quarter and Full Year 2023 Earnings

*February 22, 2024*



**BIOMARIN**<sup>®</sup>

*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 about: the expectations of Total Revenues, Non-GAAP Operating Margin Percentage, and Non-GAAP Diluted EPS for the full-year 2024; cash flows from operating activities; BioMarin's expectations regarding revenue growth potential, including the potential revenue growth opportunities of VOXZOGO in achondroplasia and new indications, ROCTAVIAN's potential to become a standard of care gene therapy, BioMarin's ability to create an annuity-like base business; BioMarin's expectations regarding profitability opportunities, including potential for greater EPS growth; BioMarin's focus on its strategic priorities, including ability to maintain VOXZOGO's momentum on achondroplasia launch and leverage broadened labels as well as the long-term potential of ROCTAVIAN, and the anticipated benefits from the same; BioMarin's plans regarding its communication approach, including with respect to the timing and content of Investor Day 2024; 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; the optimization of BioMarin's cost structure to accelerate the growth of profit margins and EPS; the commercialization of BioMarin's products; the expected benefits and availability of BioMarin's 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 February 22, 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, 2022 as such factors may be updated by any subsequent reports including BioMarin's Quarterly Report on Form 10-Q for the quarter ended September 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 of intangible assets, stock-based compensation expense, contingent consideration expense, and, in certain periods, certain other specified items, as detailed below when applicable. Non-GAAP Operating Margin percentage is defined by the company as GAAP Income from Operations, excluding amortization of intangible assets, stock-based compensation expense, contingent consideration expense, and, in certain periods, certain other specified items, divided by Total Revenues. The company also includes a Non-GAAP adjustment for the estimated tax impact of the reconciling items. 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.

# BioMarin 2024: At the Inflection Point of Opportunity

## Strong Revenue Growth Potential

- Significant multi-year growth driver with VOXZOGO® in achondroplasia and new indications
- ROCTAVIAN™ for severe hemophilia A as a potential standard of care gene therapy
- Durable, profitable, “IRA-insulated” annuity-like base business

## Significant Profitability Opportunities

- 8 approved products driving industry-leading revenue growth
- Financially self-sustaining as a profitable and cash flow positive enterprise
- Clear potential for greater EPS growth

## Industry-leading Innovation through Proven R&D Engine

- 100% commercialization rate for Phase 3 assets; 8 internally developed commercial products
- >50% Investigational New Drug (IND) to marketing authorization approval rate
- Multi-modal expertise, global regulatory capabilities and wholly-owned manufacturing

# Initial Priorities as Outlined in January 2024



## ACCELERATE AND MAXIMIZE THE VOXZOGO OPPORTUNITY

- VOXZOGO indication expansion is **the** top priority for BioMarin
- Maintain momentum on achondroplasia launch and leverage broadened labels



## ESTABLISH ROCTAVIAN OPPORTUNITY

- Important progress made in market access and readiness
- 2024 and 2025 will further inform ROCTAVIAN's uptake curve and long-term potential



## FOCUS R&D ON THE MOST PRODUCTIVE ASSETS

- Prioritize therapies with transformational benefits and high commercial potential
- Efficient investment through proof-of-concept with stringent criteria before advancing assets



## ACCELERATE EPS GROWTH AND EXPAND MARGINS

- Continued significant revenue growth, intense focus on operational excellence, prioritization of investments, and optimization of cost structure to accelerate the growth of profit margins and EPS

# Fourth Quarter and Full Year 2023 Key Highlights and 2024 Outlook



## **2023 Record Financial Results Were Driven by Global Demand for VOXZOGO**

Q4'23 VOXZOGO net revenues increased 118% Q/Q and FY'23 increased 178% Y/Y



## **Total Revenues of \$646.2 million in the Fourth Quarter 2023 Increased 20% Q/Q**

Produced double-digit revenue growth and significant operating leverage in 2023, with non-GAAP diluted earnings per share increasing 36% Y/Y



## **VOXZOGO Approvals for Younger Children Driving Market Expansion**

Approximately 70% of new U.S. treatment starts in Q4'23 were for children <5 years of age; 2024 growth expected to accelerate across key large markets driven by expansion into younger age groups, as well as children of all ages



## **Pivotal Program with VOXZOGO in Hypochondroplasia to Begin Treatment Study Mid-year 2024**

Enrollment in the baseline, run-in arm of the study began in the fourth quarter of 2023



## **2024 Financial Outlook Reflects Anticipated Double-digit Total Revenue Growth, Significant Non-GAAP Operating Margin Expansion and Non-GAAP EPS Growing Faster than Revenues**

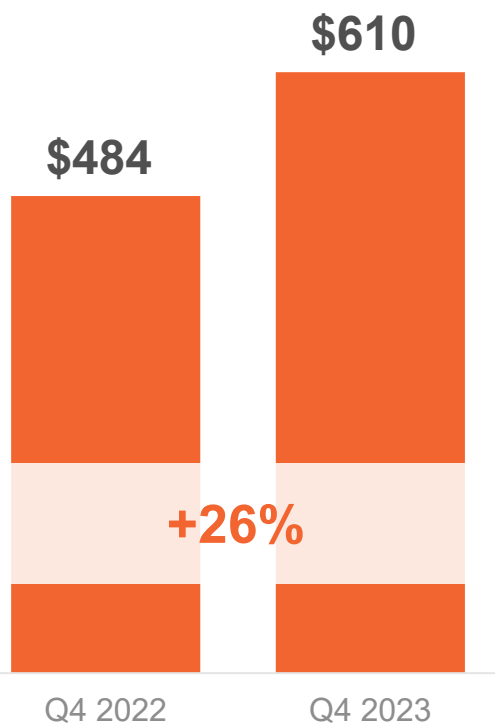
# Fourth Quarter 2023 Key Financial Metrics

(In millions, except percentage data)

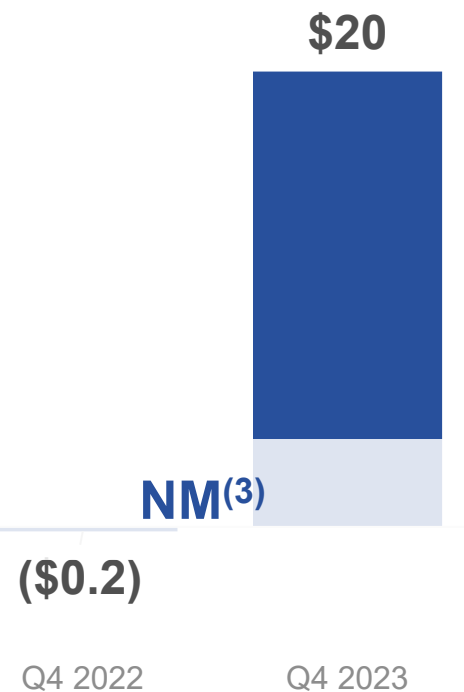
## Total Reported Revenue Growth



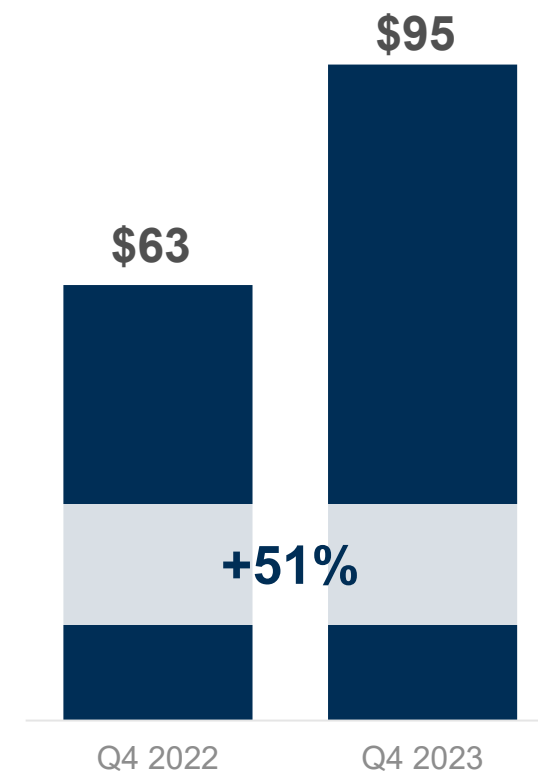
## Total Revenue Growth<sup>(1)</sup> Excluding KUVAN<sup>®</sup>



## GAAP Net Income



## Non-GAAP Income<sup>(2)</sup>



(1) Includes total revenues excluding revenues from KUVAN of \$36.7 million and \$53.6 million for the fourth quarter of 2023 and 2022, respectively.

(2) 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 13 of this presentation for reconciliations to the comparable information reported under U.S. GAAP.

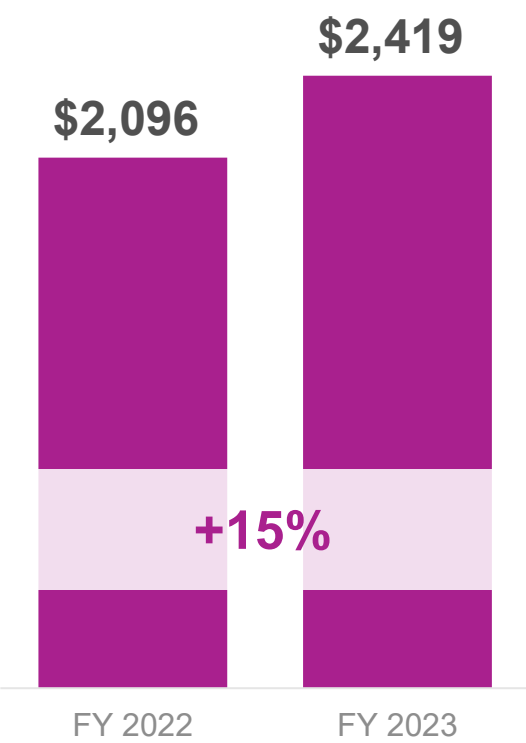
(3) NM = Not Meaningful



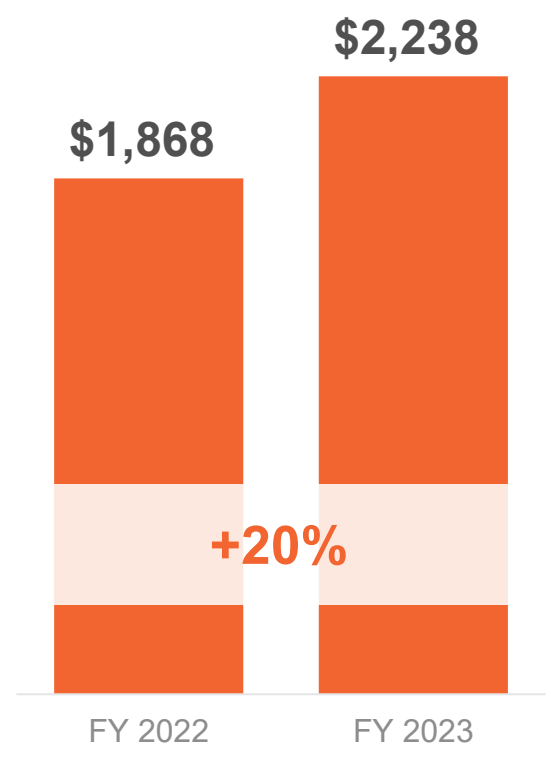
# Full Year 2023 Key Financial Metrics

(In millions, except percentage data)

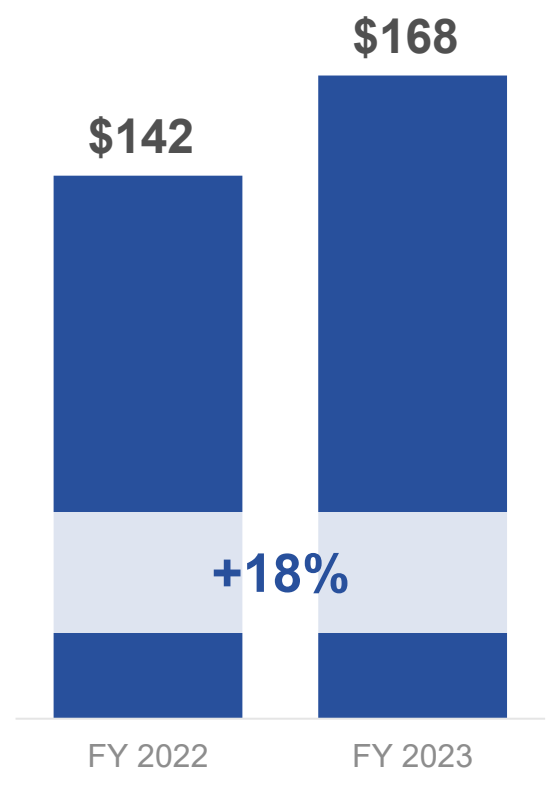
## Total Reported Revenue Growth



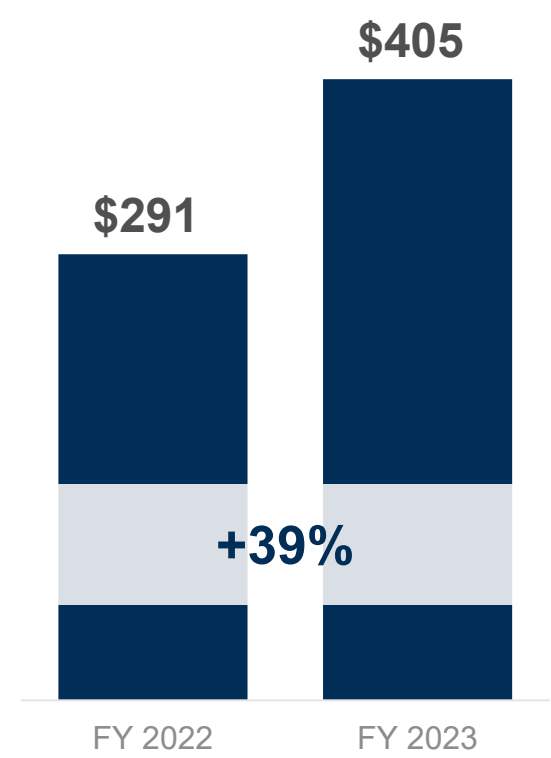
## Total Revenue Growth<sup>(1)</sup> Excluding KUVAN®



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



## Non-GAAP Income<sup>(3)</sup>



(1) Includes total revenues excluding revenues from KUVAN of \$180.8 million and \$227.6 million for full year 2023 and 2022, respectively.

(2) GAAP Net income in the twelve months ended December 31, 2022 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.

(3) 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 13 of this presentation for reconciliations to the comparable information reported under U.S. GAAP.

# Full Year 2023 Actuals Versus 2023 Guidance

<i>(In millions, except per share and percentage data)</i>	<b>2023 Guidance As of November 1, 2023</b>	<b>2023 Actuals</b>
Enzyme Products Revenue <sup>(1)</sup>	\$1,700 - \$1,775	\$1,718
ROCTAVIAN Revenue	less than \$10M	\$3.5
VOXZOGO Revenue	\$435 - \$455	\$470
<b>Total Revenues</b>	<b>\$2,390 - \$2,470</b>	<b>\$2,419</b>
Gross Margin %	77.8% - 79%	79%
R&D % of Revenue	30% - 32%	30.9%
SG&A % of Revenue	36% - 38%	38.7%
<b>GAAP Net Income <sup>(2)</sup></b>	<b>\$170 - \$210</b>	<b>\$168</b>
<b>GAAP Diluted EPS</b>	<b>\$0.85 - \$1.05</b>	<b>\$0.87</b>
<b>Non-GAAP Income <sup>(3)</sup></b>	<b>\$380 - \$410</b>	<b>\$405</b>
<b>Non-GAAP Diluted EPS <sup>(4)</sup></b>	<b>\$1.90 - \$2.05</b>	<b>\$2.08</b>

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

(2) GAAP Net Income in the twelve months ended December 31, 2022 included a \$89.0 million 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.

(3) Non-GAAP Income is defined by the company as reported GAAP Net Income (Loss), 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 beginning on page # of this press release for a complete discussion of the company's Non-GAAP financial information and reconciliations to the comparable information reported under U.S. GAAP.

(4) Non-GAAP Diluted EPS is defined by the company as Non-GAAP Income divided by Non-GAAP diluted weighted-average 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.

# Evolution of our Communications Approach in 2024 and Beyond



## Maturing Guided Line Items (4Q23 Earnings Call)

- Total Revenues
- Non-GAAP Operating Margin<sup>(1)</sup>
- Non-GAAP Earnings Per Share<sup>(2)</sup>



## 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 (3Q24)

- New leadership perspectives on company strategy and long-term aspirations
- Strategic and Operating Review Committee outcomes
- Long-term financial targets

(1) Non-GAAP Operating Margin percentage is defined by the company as GAAP Income from Operations, excluding amortization of intangible assets, stock-based compensation expense, contingent consideration expense, and, in certain periods, certain other specified items, divided by Total Revenues.

(2) Non-GAAP Diluted EPS is defined by the company as Non-GAAP Income divided by weighted-average diluted shares outstanding.

# Updated Guidance Line Items Beginning Full Year 2024

<i>(In millions, except per share and % data)</i>	<b>New Guidance</b> As of February 22, 2024
<b>Total Revenues</b>	<b>\$2,700 - \$2,800</b>
<b>Non-GAAP Operating Margin %<sup>(1)</sup></b>	<b>23% - 24%</b>
<b>Non-GAAP Diluted EPS<sup>(2)</sup></b>	<b>\$2.60 - \$2.80</b>

(1) 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 certain specified items divided by Total Revenues.

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

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

(In millions)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	2022	2023	2022
<b>GAAP Reported Net Income (Loss)</b>	<b>\$ 20.4</b>	<b>\$ (0.2)</b>	<b>\$ 167.6</b>	<b>\$ 141.6</b>
Adjustments				
Stock-based compensation expense - COS	4.5	4.3	17.6	17.7
Stock-based compensation expense - R&D	16.3	13.8	65.7	61.7
Stock-based compensation expense - SG&A	34.0	28.7	123.8	116.9
Amortization of intangible assets	15.2	15.7	62.2	62.8
Contingent consideration	—	0.6	—	4.4
Gain on sale of non-financial assets <sup>(2)</sup>	—	—	—	(108.0)
Severance and employee termination benefits <sup>(3)</sup>	—	18.2	(0.5)	23.0
Asset impairments <sup>(4)</sup>	14.0	—	14.0	—
Loss on investments <sup>(5)</sup>	11.9	—	24.5	—
Income tax effect of adjustments	(21.4)	(18.0)	(69.5)	(29.3)
<b>Non-GAAP Income</b>	<b>\$ 94.9</b>	<b>\$ 63.1</b>	<b>\$ 405.4</b>	<b>\$ 290.8</b>

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

(2) 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.

(3) Represents severance and employee termination benefit charges and subsequent adjustments included in SG&A related to the company's organizational redesign announced in the fourth quarter of 2022.

(4) Represents the write-off of capitalized tooling and fixed assets in SG&A associated with the company's decision to cease development of the current VOXZOGO pen device in the fourth quarter of 2023.

(5) Represents the impairment losses on an investment in non-marketable equity securities and a convertible note recorded in Other income (expense), net in the first and fourth quarter of 2023, respectively.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	2022	2023	2022
<b>GAAP Diluted EPS</b>	<b>\$ 0.11</b>	<b>\$ (0.00)</b>	<b>\$ 0.87</b>	<b>\$ 0.75</b>
Adjustments				
Stock-based compensation expense	0.27	0.24	1.04	1.02
Amortization of intangible assets	0.08	0.08	0.31	0.33
Contingent consideration	—	—	—	0.02
Gain on sale of non-financial assets <sup>(2)</sup>	—	—	—	(0.56)
Severance and employee termination benefits <sup>(3)</sup>	—	0.09	—	0.12
Asset impairments <sup>(4)</sup>	0.07	—	0.07	—
Loss on investments <sup>(5)</sup>	0.06	—	0.12	—
Income tax effect of adjustments	(0.10)	(0.09)	(0.33)	(0.15)
<b>Non-GAAP Diluted EPS</b>	<b>\$ 0.49</b>	<b>\$ 0.33</b>	<b>\$ 2.08</b>	<b>\$ 1.53</b>

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

(2) 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.

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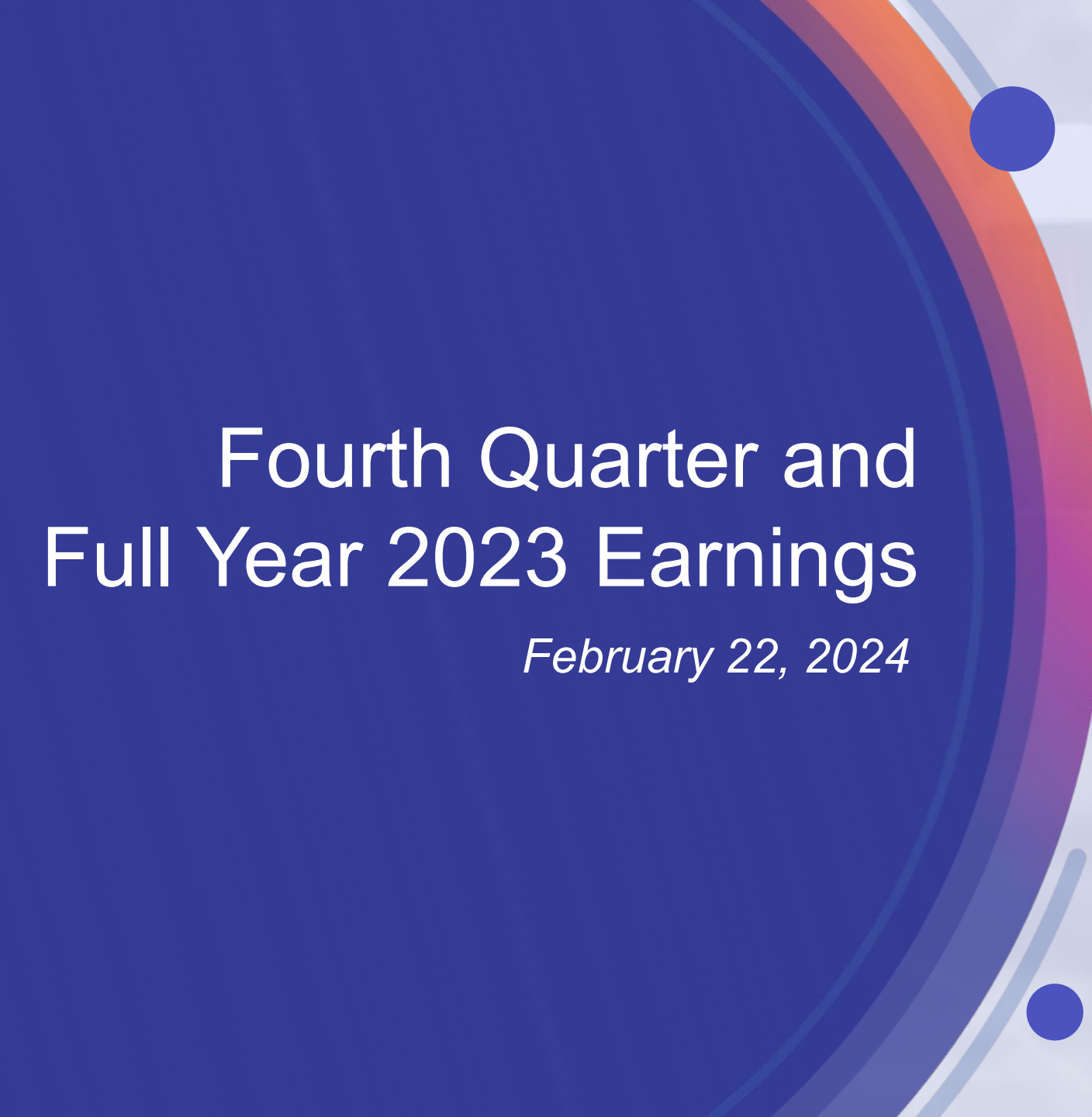
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(5) Represents the impairment losses on an investment in non-marketable equity securities and a convertible note recorded in Other income (expense), net in the first and fourth quarter of 2023, respectively.

# Reconciliation of GAAP to Non-GAAP Weighted-Average Shares Outstanding

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	2022	2023	2022
<b>GAAP Weighted-Average Dilutive Shares Outstanding</b>	<b>191.8</b>	<b>186.0</b>	<b>191.6</b>	<b>189.0</b>
Adjustments				
Common stock issuable under the company's equity plans	—	4.1	—	—
Common stock issuable under company's convertible debt <sup>(1)</sup>	8.4	4.0	8.4	4.0
<b>Non-GAAP Weighted-Average Dilutive Shares Outstanding</b>	<b>200.2</b>	<b>194.1</b>	<b>200.0</b>	<b>193.0</b>

(1) Common stock issuable under the company's convertible debt was excluded from the computation of GAAP Weighted-Average Dilutive Shares Outstanding when they were anti-dilutive. If converted, the company would issue 4.0 million shares under the convertible notes due in 2024 and 4.4 million shares under the convertible notes due in 2027.



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