

# Fourth Quarter 2024 Earnings

*February 19, 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 double-digit revenue and 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 potential of BioMarin's Enzyme Therapies portfolio, including PALYNZIQ, and the expectation regarding the full realization of the benefits of BioMarin's cost transformation program and the integration of efficiencies; 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; expectations regarding the Revenue Compound Annual Growth Rate (CAGR) of VOXZOGO and Enzyme Therapies for future periods; 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, and expected early clinical data read-outs in 2025 for BMN 333 and BMN 351; (ii) expected results from Phase 3 PALYNZIQ study for the treatment of adolescents with phenylketonuria between the ages of 12 and 17 in 2025 and plans to submit applications to expand age eligibility in the U.S. and Europe in the second half of 2025; (iii) plans for the pivotal study in hypochondroplasia to complete enrollment in the first half of 2025 and launch in 2027; (iv) the expectations regarding global expansion of VOXZOGO and that VOXZOGO will be available in more than 60 countries by 2027; (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 February 19, 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, 2023 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, 2024. 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.

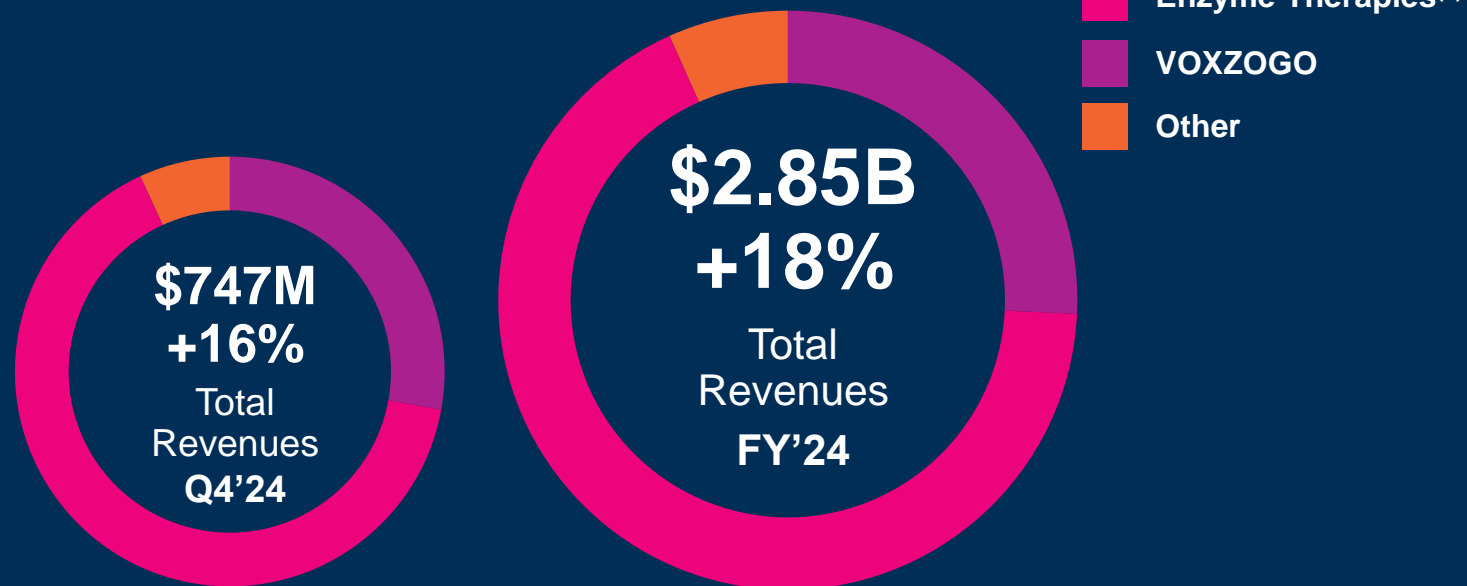
BioMarin regularly uses both GAAP and Non-GAAP results and expectations internally to assess its financial operating performance and evaluate key business decisions related to its principal business activities: the discovery, development, manufacture, marketing and sale of innovative biologic therapies.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

# BioMarin's Q4'24 and FY'24 performance drove strong revenue growth and rapid profitability expansion; BioMarin on track to multiyear growth outlook



	VOXZOGO Revenues	Enzyme Therapies <sup>(1)</sup> Revenues	GAAP Operating Margin	Non-GAAP Operating Margin <sup>(2)</sup>	GAAP Diluted Earnings per Share (EPS)	Non-GAAP Diluted EPS <sup>(2)</sup>
<b>Fourth Quarter 2024 Results</b>	<b>\$208M</b> +42%	<b>\$488M</b> +9%	<b>21.6%</b> +17.4 ppts	<b>31.1%</b> +13.9 ppts	<b>\$0.64</b> +482%	<b>\$0.92</b> +88%
<b>Full Year 2024 Results</b>	<b>\$735M</b> +56%	<b>\$1.93B</b> +12%	<b>17.0%</b> +9.3 ppts	<b>28.6%</b> +9.2 ppts	<b>\$2.21</b> +154%	<b>\$3.52</b> +69%

All growth rates are compared to the same period in 2023

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

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

# Strong Financial and Clinical Execution through Transformational 2024

## INNOVATION

- BMN 333 **first-in-human dosing ongoing**, with **initial data expected YE'25**; studies advancing with VOXZOGO in multiple indications, with hypochondroplasia pivotal study enrollment completion expected **1H'25**
- BMN 351 **proof-of-concept** data expected **mid-2025** (including dystrophin levels after 25 weeks of dosing)
- PALYNZIQ in adolescents ages 12-17 **phase 3 data** anticipated **mid-2025** to support a potential **U.S. Supplemental Biologics License Application (sBLA)** in **2H'25**

## GROWTH

- **VOXZOGO** full-year revenues increased **56% Y/Y** and global patient demand continues to grow
- **Majority** of U.S. VOXZOGO patient starts in Q4 were children **under the age of 5 years**; worldwide, as of Q4, patients were treated with VOXZOGO in **47** countries, with plans to expand to more than **60** countries by 2027
- **Enzyme Therapies** full-year revenues grew **12% Y/Y**, led by **PALYNZIQ's 17% growth** driven by increased penetration in the U.S. and Japan; Enzyme Therapies tracking to **high-single digit long-term CAGR<sup>(1)</sup>**

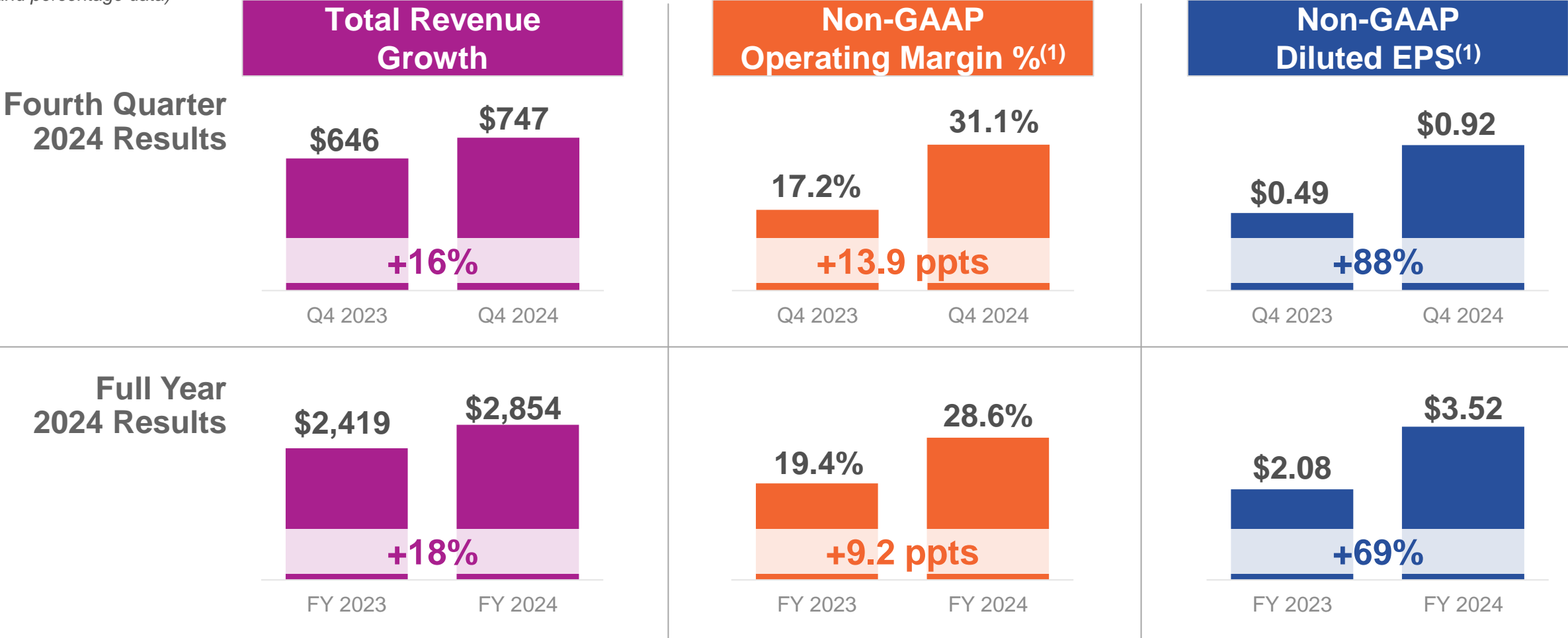
## VALUE COMMITMENT

- Strong commercial performance in 2024 delivered **record full year revenues of \$2.85B, 18% growth Y/Y**
- Top-line growth and operational execution drove 2024 Non-GAAP Operating Margin % of **28.6%** (+9.2 ppts Y/Y) and **operating cash flows of \$573M** (+260% Y/Y), supporting **reinvestment into innovation**
- **FY'25 guidance** announced for Total Revenues, Non-GAAP Operating Margin %<sup>(2)</sup>, and Non-GAAP Diluted EPS<sup>(2),(3)</sup>; on track to long-term financial guidance and outlook previously provided on September 4<sup>th</sup>, 2024

<sup>(1)</sup>CAGR = Compound Annual Growth Rate; expected Enzyme Therapies CAGR from 2023-2034; <sup>(2)</sup> Refer to slide 4 for more detail on Non-GAAP financial measures; <sup>(3)</sup>Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding.

# Q4'24 and FY'24: Significant Growth across Key Financial Metrics

(In millions, except share and percentage data)



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

# Full Year 2024 Actuals vs. Guidance

<i>(In millions, except per share and % data)</i>	<b>2024 Guidance</b> As of October 29, 2024	<b>2024 Actuals</b>
<b>Total Revenues</b>	<b>\$2,790 - \$2,825</b>	✓ <b>\$2,854</b>
<b>Non-GAAP Operating Margin %<sup>(1)</sup></b>	<b>26.5% - 27.5%</b>	✓ <b>28.6%</b>
<b>Non-GAAP Diluted EPS<sup>(1)(2)</sup></b>	<b>\$3.25 - \$3.35</b>	✓ <b>\$3.52</b>

(1) Refer to slide 4 for more detail on Non-GAAP financial measures.  
 (2) Non-GAAP Diluted EPS guidance assumed approximately 200 million Weighted-Average Diluted Shares Outstanding.



# 2025 Full Year Guidance

*BioMarin is on Course to Achieve Long-term Financial Outlook*

	2023	2024	2025	2027
Total Revenues <sup>(1)</sup>	\$2.42B	\$2.85B	\$3.1B to \$3.2B	~\$4B
Non-GAAP Operating Margin <sup>(2)</sup>	19.4%	28.6%	32% to 33%	Low-to-Mid 40%'s, starting with 40% in 2026
Non-GAAP Diluted EPS <sup>(2)(3)</sup>	\$2.08	\$3.52	\$4.20 to \$4.40	
Operating Cash Flow	\$0.2B	\$0.6B		\$1.25B+

<sup>(1)</sup>VOXZOGO contribution to full-year 2025 Total Revenues expected to be in the range of \$900 million to \$950 million. <sup>(2)</sup>Refer to slide 4 for more detail on Non-GAAP financial measures. <sup>(3)</sup>Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding.

## Appendix

### **Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information**

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

	Three Months Ended December 31,				Twelve Months Ended December 31,			
	Percent of GAAP Total Revenue		Percent of GAAP Total Revenue		Percent of GAAP Total Revenue		Percent of GAAP Total Revenue	
	2024	2023	2024	2023	2024	2023	2024	2023
<b>GAAP Income from Operations</b>	<b>\$ 161</b>	<b>21.6 %</b>	<b>\$ 27</b>	<b>4.2 %</b>	<b>\$ 484</b>	<b>17.0 %</b>	<b>\$ 186</b>	<b>7.7 %</b>
Adjustments								
Stock-based compensation expense	51	6.8	55	8.5	202	7.1	207	8.6
Amortization of intangible assets	10	1.3	15	2.3	43	1.5	62	2.6
Gain on sale of nonfinancial assets <sup>(2)</sup>	—	—	—	—	(10)	(0.4)	—	—
Severance and restructuring costs <sup>(3)</sup>	10	1.3	—	—	96	3.4	(1)	—
Asset impairments <sup>(4)</sup>	—	—	14	2.2	—	—	14	0.6
<b>Non-GAAP Income from Operations</b>	<b>\$ 232</b>	<b>31.1 %</b>	<b>\$ 111</b>	<b>17.2 %</b>	<b>\$ 815</b>	<b>28.6 %</b>	<b>\$ 469</b>	<b>19.4 %</b>

(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 represent severance and restructuring costs related to the company's 2024 portfolio strategy review and the associated organizational redesign efforts announced in the second and third quarters of 2024. These amounts also include impairments of certain right-of-use and fixed assets.

(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 first generation VOXZOGO pen device in the fourth quarter of 2023.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2024	2023	2024	2023
<b>GAAP Diluted EPS</b>	<b>\$ 0.64</b>	<b>\$ 0.11</b>	<b>\$ 2.21</b>	<b>\$ 0.87</b>
Adjustments				
Stock-based compensation expense	0.26	0.27	1.03	1.04
Amortization of intangible assets	0.05	0.08	0.22	0.31
Gain on sale of nonfinancial assets <sup>(2)</sup>	—	—	(0.05)	—
Severance and restructuring costs <sup>(3)</sup>	0.05	—	0.49	—
Asset impairments <sup>(4)</sup>	—	0.07	—	0.07
Loss on investments <sup>(5)</sup>	—	0.06	0.03	0.12
Income tax effect of adjustments	(0.08)	(0.10)	(0.39)	(0.33)
<b>Non-GAAP Diluted EPS</b>	<b>\$ 0.92</b>	<b>\$ 0.49</b>	<b>\$ 3.52</b>	<b>\$ 2.08</b>

(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 represent severance and restructuring costs related to the company's 2024 portfolio strategy review and the associated organizational redesign efforts announced in the second and third quarters of 2024. These amounts also include impairments of certain right-of-use and fixed assets.

(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 first generation VOXZOGO pen device in the fourth quarter of 2023.

(5) The current period represents a downward adjustment to non-marketable equity securities recorded in Other income (expense), net in the first quarter of 2024. The prior year represents the impairment losses on an investment in non-marketable equity securities and a convertible note recorded in Other expense, net in the first and fourth quarter of 2023, respectively.

# Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2024	2023	2024	2023
<b>GAAP Weighted-Average Diluted Shares Outstanding</b>	<b>196.6</b>	<b>191.8</b>	<b>196.7</b>	<b>191.6</b>
Adjustments				
Common stock issuable under the company's convertible debt <sup>(1)</sup>	—	8.4	—	8.4
<b>Non-GAAP Weighted-Average Diluted Shares Outstanding</b>	<b>196.6</b>	<b>200.2</b>	<b>196.7</b>	<b>200.0</b>

(1) Common stock issuable under the company's convertible debt was excluded from the computation of GAAP Weighted-Average Diluted Shares Outstanding when they were anti-dilutive. If converted, for the prior year comparative period, the company would have issued approximately 4.4 million shares under the convertible notes due in 2027 and 4 million shares under the convertible notes that matured on August 1, 2024.