Second Quarter 2025 Earnings

August 4, 2025

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 increasing growth and increasing operating expenses in the remainder of 2025, and the underlying drivers of those results, such as the revenue opportunity represented by treatments for Skeletal Conditions, namely VOXZOGO and VOXZOGO's contribution to full-year 2025 Total Revenues, the expected demand and continued growth of BioMarin's Enzyme Therapies portfolio, the anticipated benefits of BioMarin's acquisition of Inozyme Pharma, Inc. and the accounting treatment of such acquisition; 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, BMN 351 and BMN 401 (formerly INZ-701), the anticipated initial data read-out for BMN 351 by year-end, the expected Phase 2 study for BMN 349 in the first half of 2026, the anticipated initial readout for the BMN 401 ENERGY 3 study in the first quarter of 2026 and potential launch in 2027, plans to advance BMN 401 for the treatment of ENPP1 deficiency across additional age groups as well as potential use in other indications, 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 for a potential launch in 2030; (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, with potential approval and launch in 2026; (iii) expected topline data from the VOXZOGO pivotal study in hypochondroplasia in 2026 and plans to submit applications in 2026 for a potential launch in 2027; (iv) the expectations regarding higher VOXZOGO revenue in the second half of 2025 compared to the first half of 2025 and the underlying assumptions for such expectations; and (v) plans to advance five new VOXZOGO indications with BioMarin's CANOPY clinical program; the expectation that any new tariffs would have limited impact in 2025; expectations for BMN 333's efficacy compared to VOXZOGO's and ability to set a new standard of treatment for achondroplasia; plans to expand VOXZOGO in more than 60 countries by 2027; 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 August 4, 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, 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 R&D expenses and Non-GAAP SG&A expenses are defined by the company as GAAP R&D expenses and GAAP SG&A expenses, respectively, excluding stock-based compensation 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 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. 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.

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 R&D expenses, Non-GAAP SG&A expenses, 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 press release and the accompanying tables 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 23 for the reconciliations to the comparable information reported under U.S. GAAP.

Q2’25 Update:

Alexander Hardy, President and CEO

- Key Business Updates

Brian Mueller, Chief Financial Officer

- Financial Results

Cristin Hubbard, Chief Commercial Officer

- Commercial Update

Greg Friberg, Chief R&D Officer

- R&D Update

Q&A

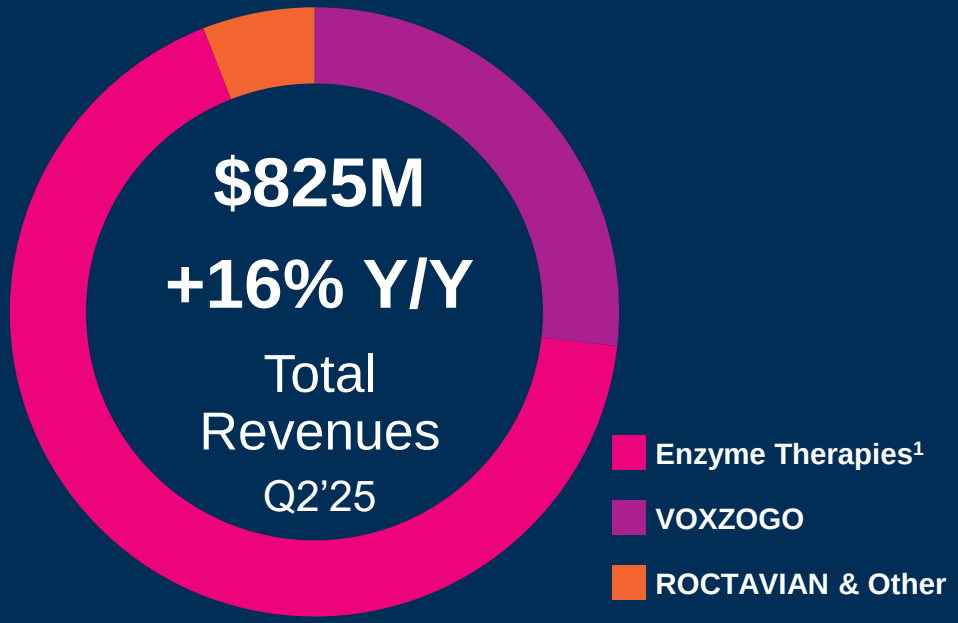
Q2'25 Key Business Updates

Alexander Hardy

President and Chief Executive Officer

Strong Q2 Performance Across All Aspects of the Business

Strong Q2'25 revenue growth and **significant profitability expansion** driven by **execution across the business**



INNOVATION

- **BMN 333** Phase 1 data showed **>3x** (AUC PK[2]) free CNP levels vs. other long-acting CNP[3]
- **Inozyme acquisition completed July 1, 2025**, strengthening Enzyme Therapies portfolio with **BMN 401** (formerly INZ-701)

GROWTH

- **Strong Q2 revenue** led by **VOXZOGO** (+20% Y/Y), **PALYNZIQ** (+20% Y/Y), and **VIMIZIM** (+21% Y/Y)

VALUE COMMITMENT

- Q2 GAAP EPS and Non-GAAP EPS[4] expanded at **multiple times** the rate of revenue growth, Y/Y
- Expect **business development** opportunities to augment BioMarin's growth

All growth rates are compared to second quarter 2024

[1]Enzyme Therapies include ALDURAZYME, BRINEURA, NAGLAZYME, PALYNZIQ, and VIMIZIM; [2]AUC PK = area under the curve pharmacokinetic; [3]Breinholt et al., Br J Clin Pharmacol. 2022;88(12):5203–5215. doi:10.1111/bcp.15369

[4]Refer to slide 3 for more detail on Non-GAAP financial measures

BioMarin's Strong 2H 2025 Outlook

Executing on Key Priorities to Deliver Significant Value Creation in 2025

Continued Strong **Growth**

Progressing Prioritized **Pipeline** Assets

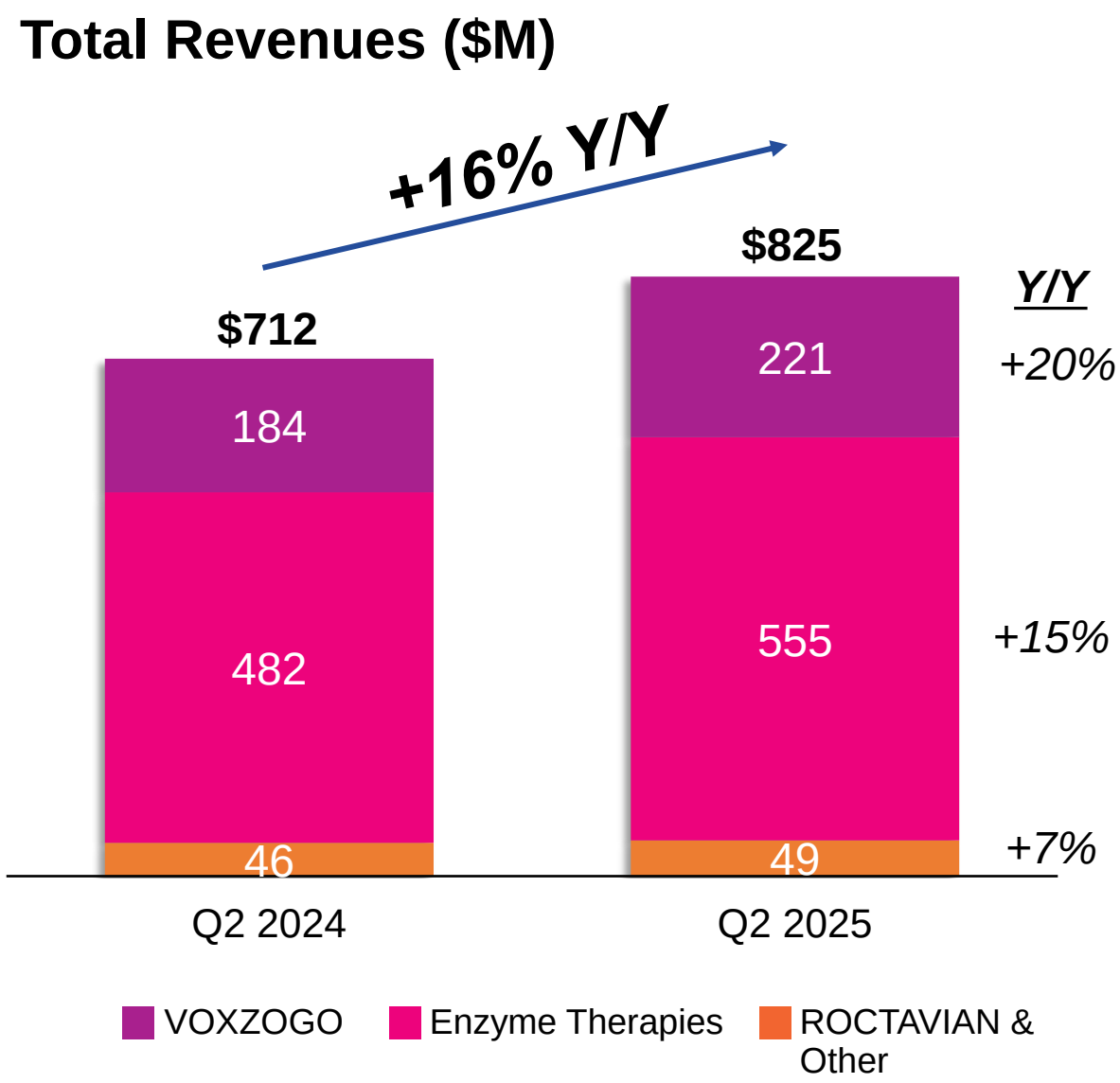
Augment Growth with **Business Development**

Q2’25 Financial Results

Brian Mueller

Executive Vice President, Chief Financial Officer

Double-digit Top-line Growth in Q2'25 Y/Y



VOXZOGO

- Y/Y growth fueled by strong global expansion
- 2H'25 poised to deliver higher revenue vs. 1H'25
- Expect **$900M to $935M** revenue in FY'25

Enzyme Therapies

- Y/Y growth reflective of strong demand and order timing from key regions
- PALYNZIQ and VIMIZIM strong contributors to Q2 growth

ROCTAVIAN

- $9M Q2 revenue
- Q2 contributions led by U.S. and Italy

FY'25 Total Revenues guidance increased to **$3,125M** to **$3,200M**

Operating Expenses Expected to Increase in 2H'25 vs. 1H'25

Q2 2025 Results

In millions, except percentages	Q2'25	Y/Y Change
GAAP R&D	$161	(12%)
GAAP SG&A	$232	(12%)
GAAP Operating Margin	33.5%	+16.6 ppts
Non-GAAP R&D[1]	$147	(14%)
Non-GAAP SG&A[1]	$203	+5%
Non-GAAP Operating Margin[1]	39.9%	+8.7 ppts

[1]Refer to slide 3 for more detail on Non-GAAP financial measures

Non-GAAP R&D and SG&A Expenses

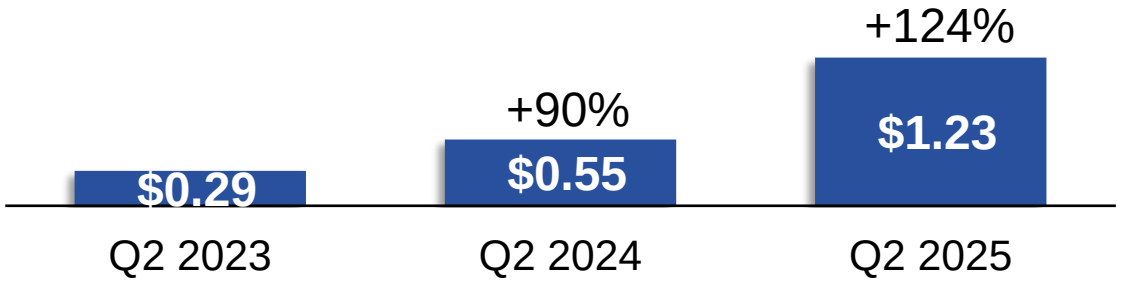
- R&D decreased Y/Y, benefiting from focused R&D investment following 2024 strategic review
- SG&A increased Y/Y to support enterprise resource planning system implementation and business unit expansion initiatives
- 2H'25 operating expenses expected to increase vs. 1H'25 as clinical programs and commercial initiatives advance

Non-GAAP Operating Margin

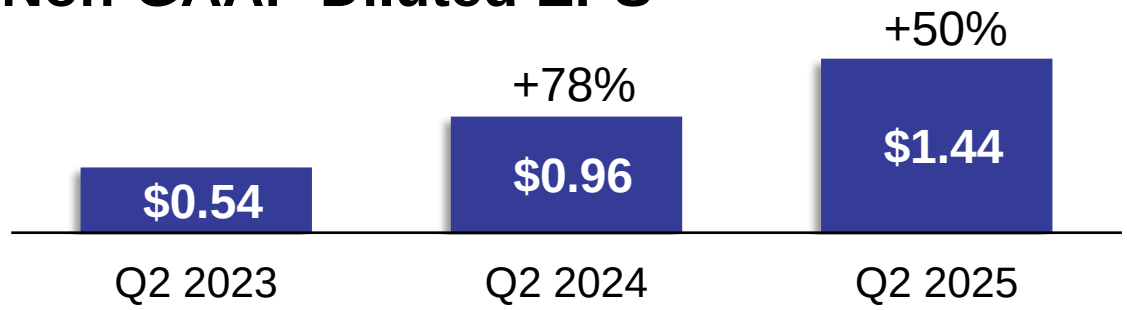
- Lower Q2 operating expenses Y/Y combined with double-digit revenue growth resulted in increased Q2 operating margin
- Higher anticipated operating expenses in 2H'25 are expected to decrease 2H'25 operating margin compared to 1H'25
- FY'25 guidance updated to **33%** to **34%**

Increasing Profitability Driving Cash Flow and Innovation Expansion

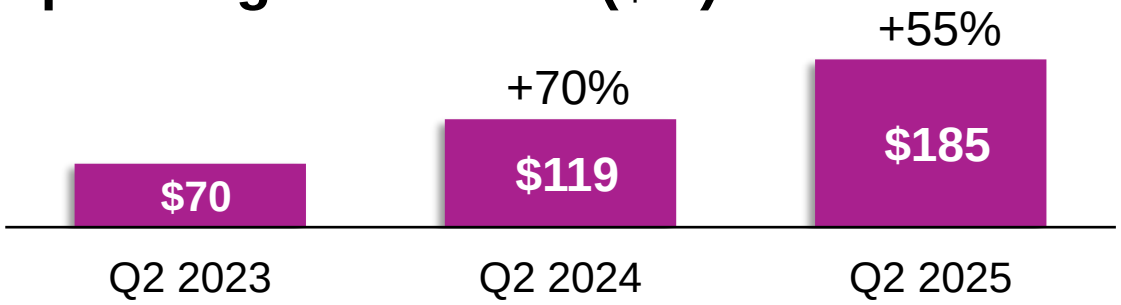
GAAP Diluted EPS



Non-GAAP Diluted EPS[1]



Operating Cash Flow ($M)



Non-GAAP Diluted EPS

- Q2 results increased at **>3x** the rate of revenue growth, Y/Y, reflecting implementation of operational efficiencies
- EPS expected to decrease in future quarters in 2025 due to increased business unit investments
- FY'25 guidance updated to **$4.40 to $4.55**

Operating Cash Flow

- Growing profitability generated significant Q2 cash flow, **+55%** Y/Y
- Increasing operating cash flow expected to continue, supporting priority of investment in innovation and future growth

Percentages reflect year-over-year change; [1]Refer to slide 3 for more detail on Non-GAAP financial measures

Updated Full-year 2025 Guidance

(In millions, except per share and % data)	Prior Guidance As of May 1, 2025	Updated Guidance As of August 4, 2025
Total Revenues[1]	**$3,100 to $3,200**	**$3,125 to $3,200**
Non-GAAP Operating Margin %[2]	**32% to 33%**	**33% to 34%**
Non-GAAP Diluted EPS[2,3]	**$4.20 to $4.40**	**$4.40 to $4.55**

Guidance excludes impact of acquisition of Inozyme Pharma, which closed July 1, 2025

- BioMarin expects to account for the Inozyme acquisition as an asset purchase and record the impact of acquired in-process research and development (IPR&D) charges in its third quarter 2025 financial results[4]
- The company expects to provide an update on full-year 2025 guidance items, including impact of the acquired IPR&D charges, in its third quarter 2025 earnings update

Reaffirmed guidance reflects the impact of tariffs, and BioMarin expects that any new tariffs would have a limited impact for 2025. [1]VOXZOGO contribution to full-year 2025 Total Revenues expected to be in the range of $900 million to $935 million. [2]Refer to slide 3 for more detail on Non-GAAP financial measures; [3]Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding. [4]Financial results are subject to BioMarin's financial statement closing procedures. While acquired IPR&D charges may be incurred upon execution of acquisitions, collaborations, licensing agreements, and other business development transactions, BioMarin does not forecast acquired IPR&D charges due to the uncertainty of the future occurrence, magnitude, and timing of these transactions in any given period.

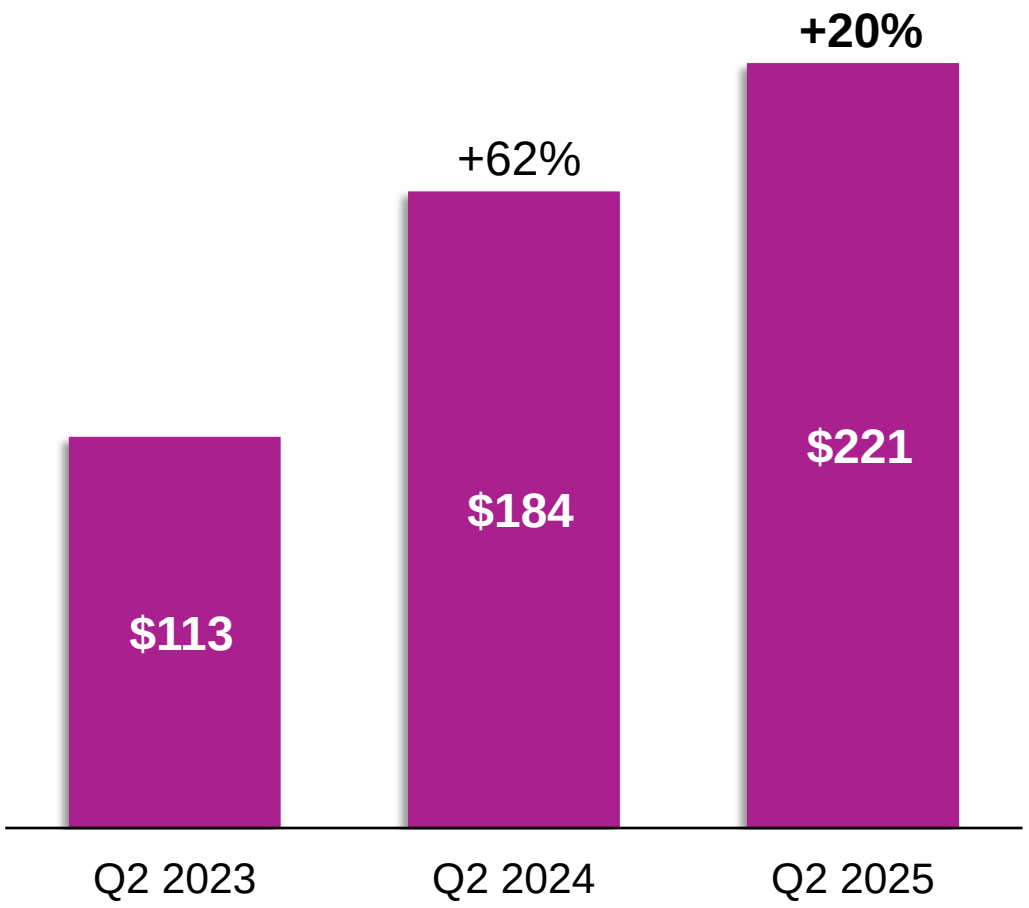
Q2’25 Commercial Update

Cristin Hubbard

Executive Vice President, Chief Commercial Officer

VOXZOGO: Strong Q2 Contributions; Global Expansion Continues

VOXZOGO® Q2 Revenue ($M)



Percentages reflect year-over-year change

Q2 Y/Y revenue growth driven by strong worldwide demand

- As of Q2, children across **51** countries had access to VOXZOGO treatment for achondroplasia (ACH)
- Targeting access in more than **60** countries by 2027
- Y/Y revenue growth in Q2 led by U.S. contributions, supported by meaningful new starts in the 0-4 year age group
- OUS Q2 expansion driven by deeper penetration in previously opened markets and incremental contributions from newly added countries
- Increasing new patient starts, momentum from U.S. initiatives, and ongoing OUS expansion expected to drive higher revenue in 2H'25 vs. 1H'25
- 2H'25 revenue expected to be weighted to Q4

Next Potential VOXZOGO Indication: Hypochondroplasia (HCH)

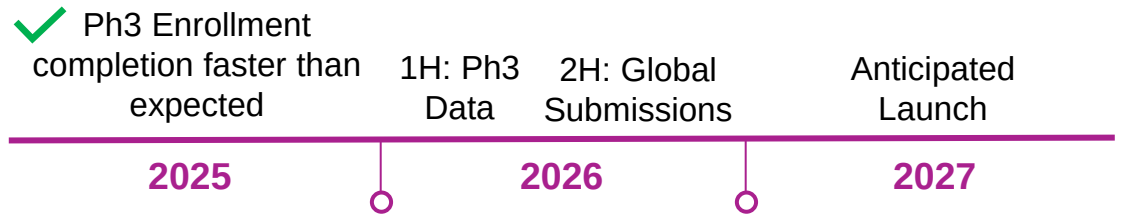
VOXZOGO Profile to Address HCH

- HCH is a skeletal condition with significant unmet need; no genetically targeted therapies
- **Hypochondroplasia characteristics:**
  - On average, children with hypochondroplasia are approximately two to three standard deviations below average height
  - Comorbidities can present as combination of disproportionality, macrocephaly, ENT and/or musculoskeletal related issues
- VOXZOGO proof-of-concept in HCH demonstrated in Phase 2 (1.81 cm/year AGV increase)

Leveraging Global ACH Experience

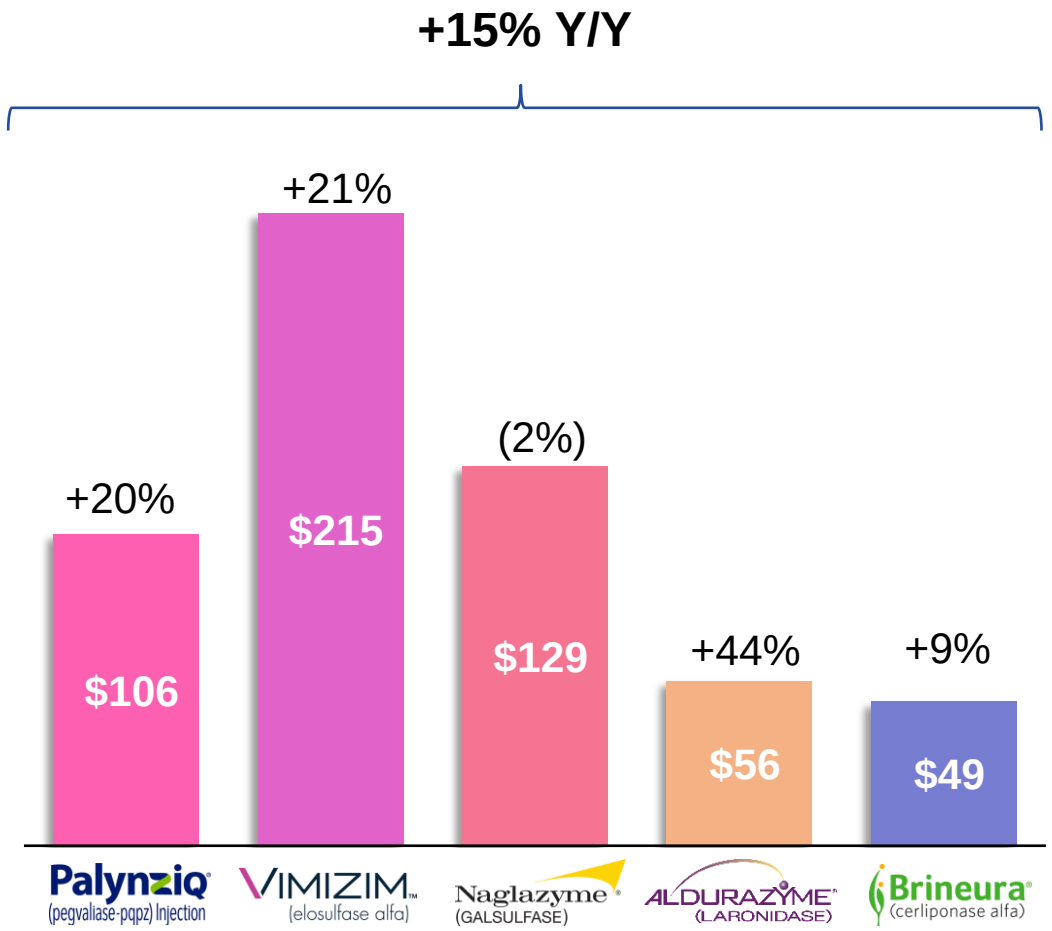
- Engaging with Skeletal Conditions medical community to advance disease awareness and management
- Engaging achondroplasia KOLs, advocacy groups, and academic institutions to improve early HCH diagnosis to inform VOXZOGO treatment
- Global Skeletal Conditions commercial infrastructure to support seamless launch, should data be supportive

Next Steps



Enzyme Therapies: Q2 ↑15% Y/Y Led by PALYNZIQ and VIMIZIM

Enzyme Therapies Q2 Revenue ($M)



Strong Growth Across Enzyme Therapies

- PALYNZIQ **20%** Y/Y growth driven by greater numbers of patients titrating to daily maintenance dose and strong adherence
- VIMIZIM **21%** Y/Y growth due to ongoing global demand and large orders outside of the U.S.
- 2H'25 Enzyme Therapies revenue expected to remain robust, despite typical quarter-to-quarter order timing dynamics

Q2’25 Research & Development Update

Greg Friberg

Executive Vice President, Chief Research & Development Officer

New Today: BMN 333 Achieves Free CNP Target in PK Study

BMN 333 for Multiple Skeletal Conditions

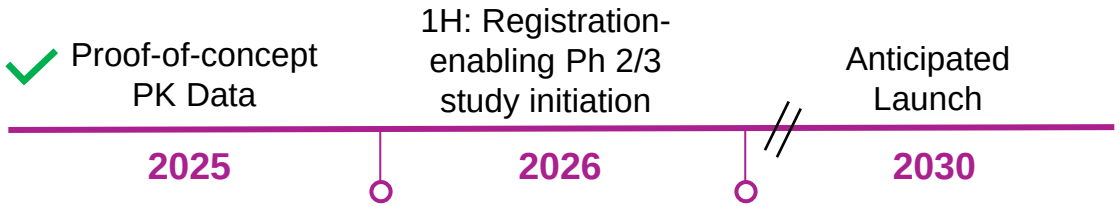
Healthy volunteer Phase 1 study with BMN 333 demonstrated free CNP levels **>3x** (area-under-the-curve) those published for other long-acting agents[1]

- Preclinical data suggest that increasing exposure of continuously delivered CNP can further increase skeletal growth
- Totality of data (preclinical and clinical) suggest that AUCs ≥3x that which has been studied with other agents could unlock a best in-class CNP profile for achondroplasia
- No safety signals observed

Go-Criteria for Phase 2/3 Reached

BMN 333 continues to advance to **registration-enabling Ph 2/3** study, expected to begin **1H'26**

- Planned study to allow for de-risking of superiority goal in a dose-ranging Phase 2 portion prior to progression to a Phase 3 comparative effectiveness versus VOXZOGO



[1]Breinholt et al., Br J Clin Pharmacol. 2022;88(12):5203–5215. doi:10.1111/bcp.15369

The Unique Benefits of PALYNZIQ to the Adolescent Population

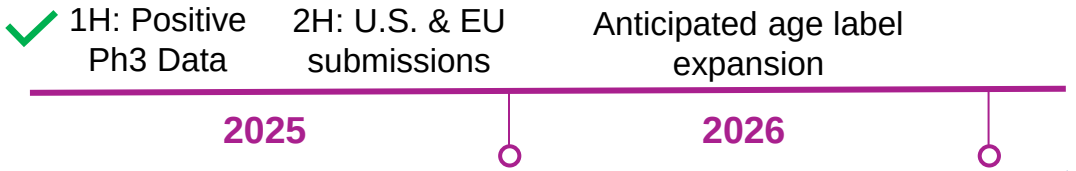
Palynziq® for phenylketonuria (PKU)

As the only **enzyme substitute therapy for PKU,** PALYNZIQ has demonstrated the ability to lower blood Phe to levels within the normal physiologic range, and to allow the possibility for an unrestricted diet, regardless of patient phenotype

PALYNZIQ Phase 3 Results[1] in Adults	
Study Phase / Duration	Phase 3 PRISM: OLE out to 36 months
Target Population Phe Levels (µmol/L)	Phe >600 on current Tx
Baseline Phe	1,233
Absolute Phe Change vs. Baseline	-662 at 12 months (n=164) -882 at 24 months (n=89)
Dietary Goals	Unrestricted Diet

Palynziq® for adolescents

- Submissions for 12 to 17 year age cohort in U.S. and Europe planned for **2H'25**
- Full data will be presented at a scientific congress in **2H'25**
- Caregiver support during titration period with PALYNZIQ may drive adherence during adulthood
- Potential for PALYNZIQ-treated adolescents to benefit from reduced need for medical food and increases in native protein intake



[1]PALYZIQ European public assessment report (Summary of Product Characteristics); https://www.ema.europa.eu/en/medicines/human/EPAR/palynziq

BMN 401: Potential First-in-Disease Treatment for ENPP1 Deficiency

BMN 401 Advancing in Clinic

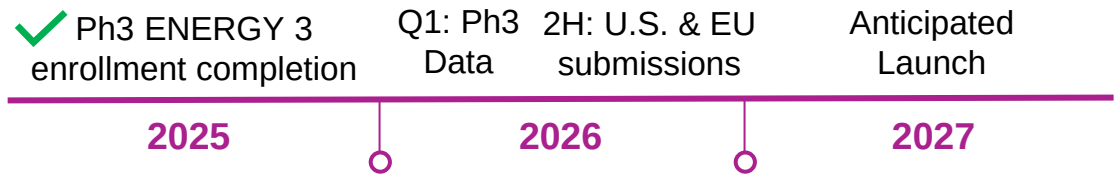
BMN 401 Phase 3 (ENERGY 3) trial ongoing for the treatment of children ages 1 to 12 years with ENPP1 Deficiency; **pivotal data expected in 1H'26,** with **potential 2027 launch**

- ENPP1 Deficiency is a rare, serious, and progressive genetic condition that can affect blood vessels, soft tissues and bones
- First and only genetically-targeted medicine with potential to treat ENPP1 Deficiency
- Progressing plans to advance BMN 401 for ENPP1 Deficiency across additional age groups
- Evaluating BMN 401 for potential use in other indications

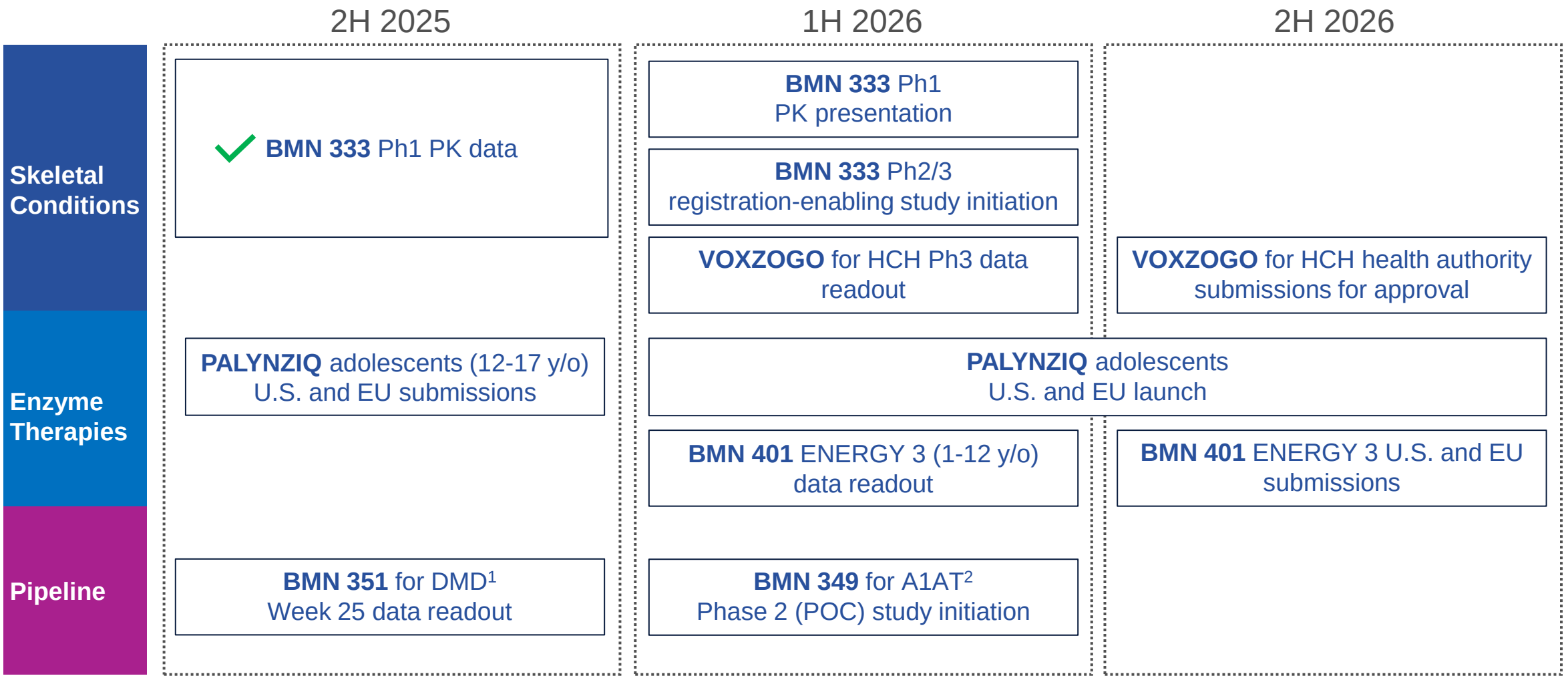
Preparation for Potential Launch

Working with patient and HCP communities to identify individuals with ENPP1 Deficiency who may benefit from BMN 401 treatment in advance of a potential launch in children age 1 to 12 years

- Strong KOL preference for a disease modifying therapy over currently limited treatment options
- Potential to address between 2-2.5k people with ENPP1 Deficiency in BioMarin's territories (includes all age groups)



Anticipated Key Pipeline Highlights over Next 1-6 Quarters



[1]DMD = Duchenne muscular dystrophy; [2]A1ATD = alpha-1 antitrypsin deficiency

Q&A

Appendix

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

Reconciliation of GAAP Reported Net Income to Non-GAAP Income[1]

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
GAAP Reported Net Income	**$ 241**	**$ 107**	**$ 426**	**$ 196**
Adjustments				
Stock-based compensation expense - COS	4	4	6	7
Stock-based compensation expense - R&D	14	13	26	34
Stock-based compensation expense - SG&A	30	31	53	66
Amortization of intangible assets	5	14	10	29
Gain on sale of nonfinancial assets [2]	—	—	—	(10)
Severance and restructuring costs [3]	—	39	—	43
Loss on investments [4]	—	5	3	5
Income tax effect of adjustments	(11)	(24)	(22)	(39)
Non-GAAP Income	$ 282	$ 189	$ 502	$ 329

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

Reconciliation of GAAP and Non-GAAP R&D and SG&A Expenses[1]

	Three Months Ended June 30,				Six Months Ended June 30,			
	2025		2024		2025		2024	
	R&D	SG&A	R&D	SG&A	R&D	SG&A	R&D	SG&A
GAAP expenses	**$ 161**	**$ 232**	**$ 184**	**$ 263**	**$ 320**	**$ 438**	**$ 389**	**$ 489**
Adjustments								
Stock-based compensation expense	(14)	(30)	(13)	(31)	(26)	(53)	(34)	(66)
Severance and restructuring costs [3]	—	—	—	(39)	—	—	—	(42)
Non-GAAP expenses	$ 147	$ 203	$ 171	$ 193	$ 294	$ 385	$ 355	$ 381

(1) Certain amounts may not sum or recalculate due to rounding.
(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 Income from Operations to Non-GAAP Income from Operations[1]

	Three Months Ended June 30,				Six Months Ended June 30,			
	2025	Percent of GAAP Total Revenue	2024	Percent of GAAP Total Revenue	2025	Percent of GAAP Total Revenue	2024	Percent of GAAP Total Revenue
GAAP Income from Operations	**$ 277**	**33.5 %**	**$ 120**	**16.9 %**	**$ 501**	**31.9 %**	**$ 209**	**15.4 %**
Adjustments								
Stock-based compensation expense	48	5.8	48	6.8	85	5.4	106	7.7
Amortization of intangible assets	5	0.6	14	2.0	10	0.6	29	2.1
Gain on sale of nonfinancial assets [2]	—	—	—	—	—	—	(10)	(0.7)
Severance and restructuring costs [3]	—	—	39	5.5	—	—	42	3.1
Non-GAAP Income from Operations	$ 329	39.9 %	$ 222	31.2 %	$ 596	37.9 %	$ 376	27.6 %

(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 Diluted EPS to Non-GAAP Diluted EPS[1]

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
GAAP Diluted EPS	**$ 1.23**	**$ 0.55**	**$ 2.19**	**$ 1.01**
Adjustments				
Stock-based compensation expense	0.24	0.24	0.43	0.53
Amortization of intangible assets	0.03	0.07	0.05	0.14
Gain on sale of nonfinancial assets [2]	—	—	—	(0.05)
Severance and restructuring costs [3]	—	0.20	—	0.21
Loss on investments [4]	—	0.02	0.02	0.02
Income tax effect of adjustments	(0.06)	(0.12)	(0.11)	(0.19)
Non-GAAP Diluted EPS [5]	$ 1.44	$ 0.96	$ 2.57	$ 1.67

(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 impairment loss on non-marketable equity securities recorded in Other income (expense), net.
(5) Non-GAAP Weighted-Average Diluted Shares Outstanding were 197.1 million and 200.5 million shares for the three months ended June 30, 2025 and 2024, respectively, and 196.6 million and 200.1 million shares for the six months ended June 30, 2025 and 2024, respectively, which were equal to the respective GAAP Weighted-Average Diluted Shares Outstanding in the periods presented.