

Fourth Quarter and Full Year 2025 Earnings

February 23, 2026

Forward-Looking Statements

This 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 2026 and future periods, and the underlying drivers of those results, such as the expected demand and continued growth of BioMarin's Enzyme Therapies portfolio, the expected growth from VOXZOGO, and the expected impact of Other Revenues; the anticipated closing and benefits of BioMarin's proposed acquisition of Amicus Therapeutics, Inc.; BioMarin's plans for investment in innovation and future growth; the timing of orders for commercial products; plans and expectations regarding the development, commercialization and commercial prospects of BioMarin's product candidates and commercial products, including the prospects and timing of actions relating to clinical studies and trials and product approvals, such as study initiations, study advancements, data readouts, submissions, filings, approvals, and label expansions; the expected benefits and availability of BioMarin's commercial products and product candidates; and potential growth opportunities and trends, including the assumptions and expectations regarding total addressable patient population (TAPP) with respect to the conditions targeted by BioMarin's product candidates and commercial products. 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 February 23, 2026, 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 September 30, 2025, as such factors may be updated by any subsequent reports. Investors 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 (Loss) 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 (Loss) 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 or 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 such Non-GAAP metrics are important internal measurements for BioMarin, BioMarin believes that providing this information in conjunction with 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. 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 18 for the reconciliations to the comparable information reported under U.S. GAAP.

Q4'25 and FY'25 Earnings Agenda:

- 1 Key Business Updates
- 2 Financial Results
- 3 Commercial Update
- 4 Research & Development Update
- 5 Q&A



Alexander Hardy
Chief Executive Officer



Brian Mueller
Chief Financial Officer



Cristin Hubbard
Chief Commercial Officer



Greg Friberg
Chief R&D Officer

Q4'25 and FY'25 Key Business Updates



Alexander Hardy
Chief Executive Officer

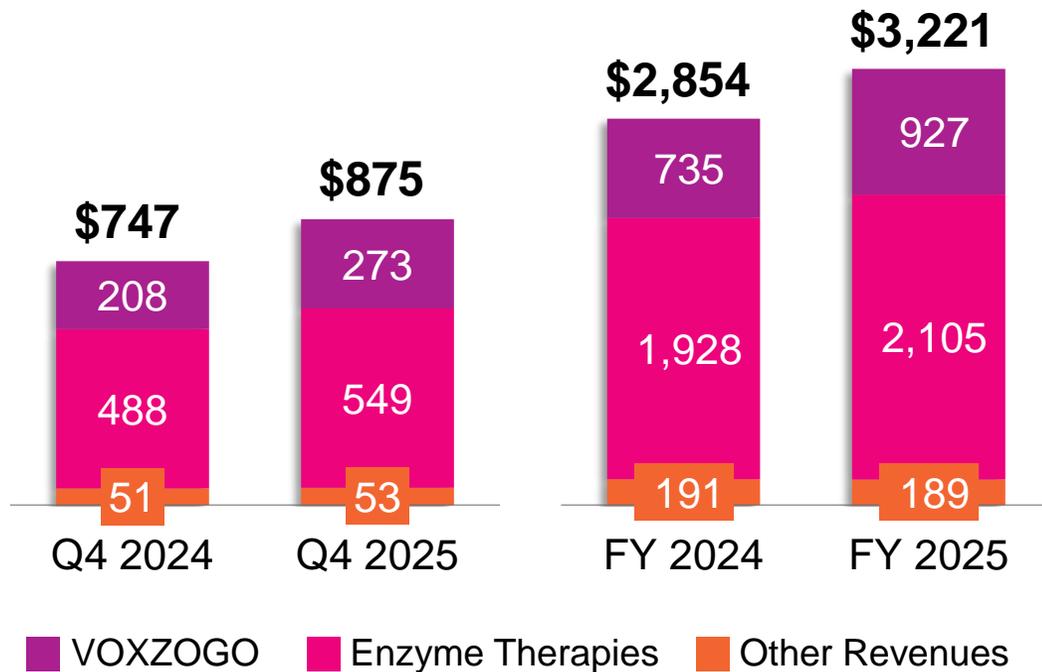
Record 2025 Performance Builds Strong Momentum into 2026

Total Revenues

\$ in millions

+17% Q4 Y/Y

+13% FY Y/Y



Anticipated Key Highlights in 2026¹

Expand Reach with New Assets

BMN 401

ENPP1 deficiency

Galafold[®]
(migalastat)

Fabry Disease

Pombiliti[™] + **Opfolda**[™]
(cipaglucosidase alfa-atga)

(miglustat) 65 mg capsules

Pompe Disease

Advance Lifecycle Innovation

VOXZOGO[®]
(vosoritide) for injection
hypochondroplasia

BMN 333
achondroplasia

Palynziq[®]
(pegvaliase-pqpz) Injection
PKU adolescents

Deliver Strong Results Across Portfolio

Palynziq[®] (pegvaliase-pqpz) Injection
Naglazyme[®] (GALSULFASE)
VIMIZIM[®] (elsulfase alfa)
Brineura[®] (cerliponase alfa)
ALDURAZYME[®] (LARONIDASE)

Galafold[®] (migalastat)
Pombiliti[™] + **Opfolda**[™]
(cipaglucosidase alfa-atga) (miglustat) 65 mg capsules

VOXZOGO[®]
(vosoritide) for injection

¹Pending closure of BioMarin's acquisition of Amicus Therapeutics (including marketed products Galafold and Pombiliti + Opfolda); acquisition is expected to close in Q2'26, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions

Q4'25 and FY'25 Financial Results

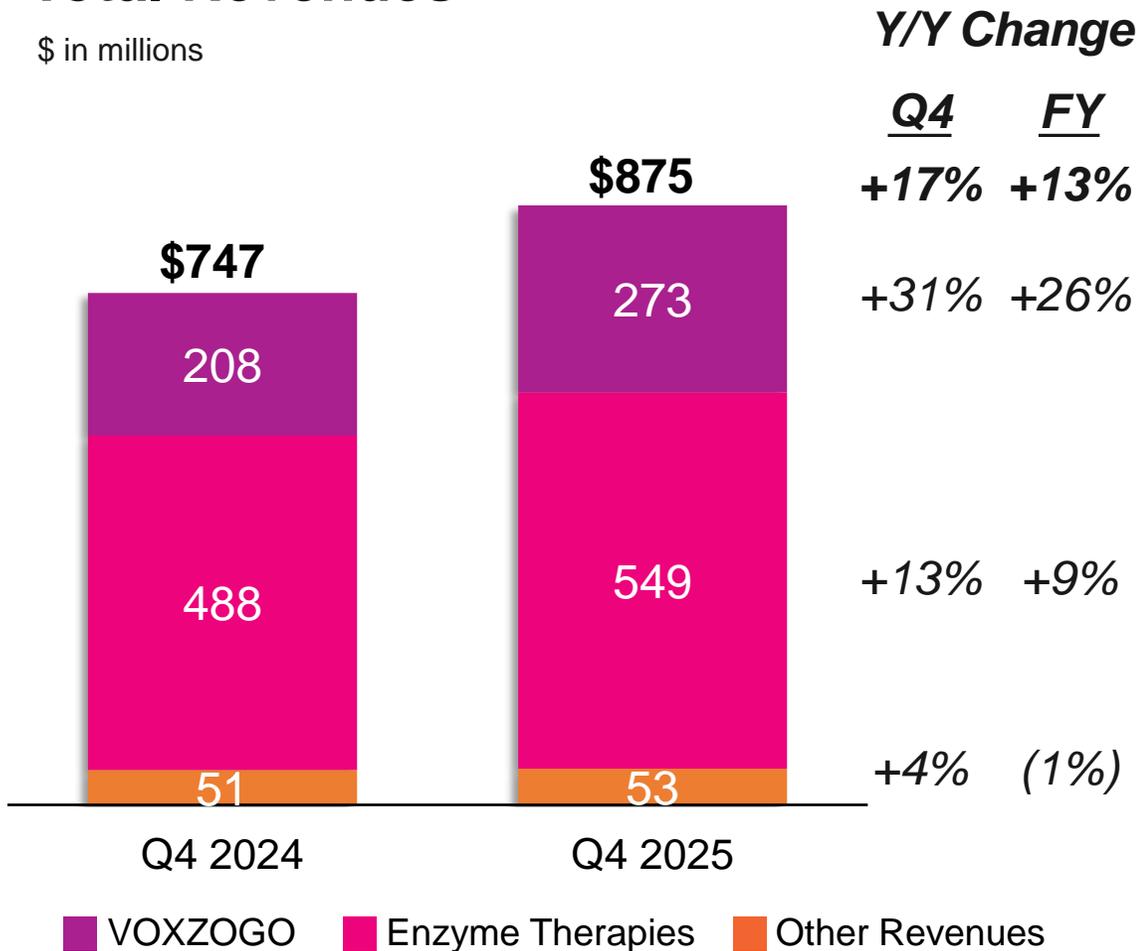


Brian Mueller
Chief Financial Officer

Q4 and FY'25 Growth Driven by Robust Global Demand across Portfolio

Total Revenues

\$ in millions



VOXZOGO

- Sustained strong global expansion; FY'25 revenue ~**73%** outside of the U.S. (OUS)
- Patient additions across all regions
- Q4'25 benefited from large OUS contracted order (not expected to repeat in Q1'26)

Enzyme Therapies

- FY'25 revenue increased **9%**, Y/Y
- PALYNZIQ: **Fourth consecutive quarter of 20% or higher** Y/Y revenue growth

FY'25 Results Reflect Strong Execution and Underlying Performance

FY 2025 Results

In millions, except percentages and per share amounts

	FY'25	Y/Y Change
GAAP R&D	\$922	+23%
GAAP SG&A	\$1,153	+14%
GAAP Operating Margin	12.7%	(4.3) pts
Non-GAAP R&D ¹	\$867	+26%
Non-GAAP SG&A ¹	\$901	+15%
Non-GAAP Operating Margin ¹	23.3%	(5.3) pts
GAAP Diluted EPS	\$1.80	(19%)
Non-GAAP Diluted EPS ¹	\$3.15	(11%)
Operating Cash Flow	\$828	+45%

ROCTAVIAN Charges Incurred in Q4'25

- **\$240M** (\$0.94 after-tax diluted EPS impact) charges on a GAAP basis due to the company's decision to voluntarily withdraw ROCTAVIAN from the market
 - **\$119M** (\$0.46 after-tax diluted EPS impact) inventory write-off reflected in GAAP and Non-GAAP results

FY'25 Non-GAAP Diluted EPS

- **\$3.15** EPS includes (\$0.46) from ROCTAVIAN inventory write-off and (\$1.10) acquired IPR&D from Inozyme acquisition
- Strong execution drove **underlying profitability expansion**

FY'25 Operating Cash Flow

- Significant operating cash flow growth continues, with **\$828M** generated in FY'25, providing capital to reinvest in innovation and future growth

¹Refer to slide 2 for more detail on Non-GAAP financial measures

Full-year 2026 Guidance

(Excludes Post-close Contributions from Amicus Acquisition¹)

	2025 Actual	2026 Guidance
<i>(In millions, except per share amounts)</i>		
Total Revenues	\$3,221	\$3,325 to \$3,425
Enzyme Therapies	\$2,105	\$2,225 to \$2,275
VOXZOGO	\$927	\$975 to \$1,025
Other Revenues	\$189	\$100 to \$125
Non-GAAP Diluted EPS^{2,3}	\$3.15	\$4.95 to \$5.15

2026 Anticipated Quarterly Dynamics

- Due to order timing, Q1'26 Total Revenues and VOXZOGO revenue expected to be similar to Q1'25
- Q1'26 Non-GAAP Diluted EPS expected to be lowest of 2026 due to revenue weighting and pre-close Amicus costs
- Enzyme Therapies and VOXZOGO: Large OUS orders to drive higher 2H revenue vs. 1H and weighted to Q4

Key Considerations

- 2026 guidance expected to be updated upon the closing of the Amicus acquisition, anticipated in Q2'26
- Total Revenues guidance reflects expectation of continued strong patient demand across Enzyme Therapies and VOXZOGO in 2026
- Other Revenue guidance reflects KUVAN, royalty revenue (including conclusion of U.S. Firdapse royalty term in January 2026), and the company's strategic decision to voluntarily withdraw ROCTAVIAN from the market
- Non-GAAP Diluted EPS guidance includes approximately \$0.25 of pre-close operating and interest expenses associated with Amicus transaction
- In 2026, excluding the impact of the Amicus transaction, Non-GAAP Operating Margin is expected to be approximately 40% for the full year

¹Post-close contributions refer to revenue, operating expense and incremental interest expense associated with acquisition financing after closing of the Amicus transaction; ²Refer to slide 2 for more detail on Non-GAAP financial measures; ³Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding.

Commercial Update

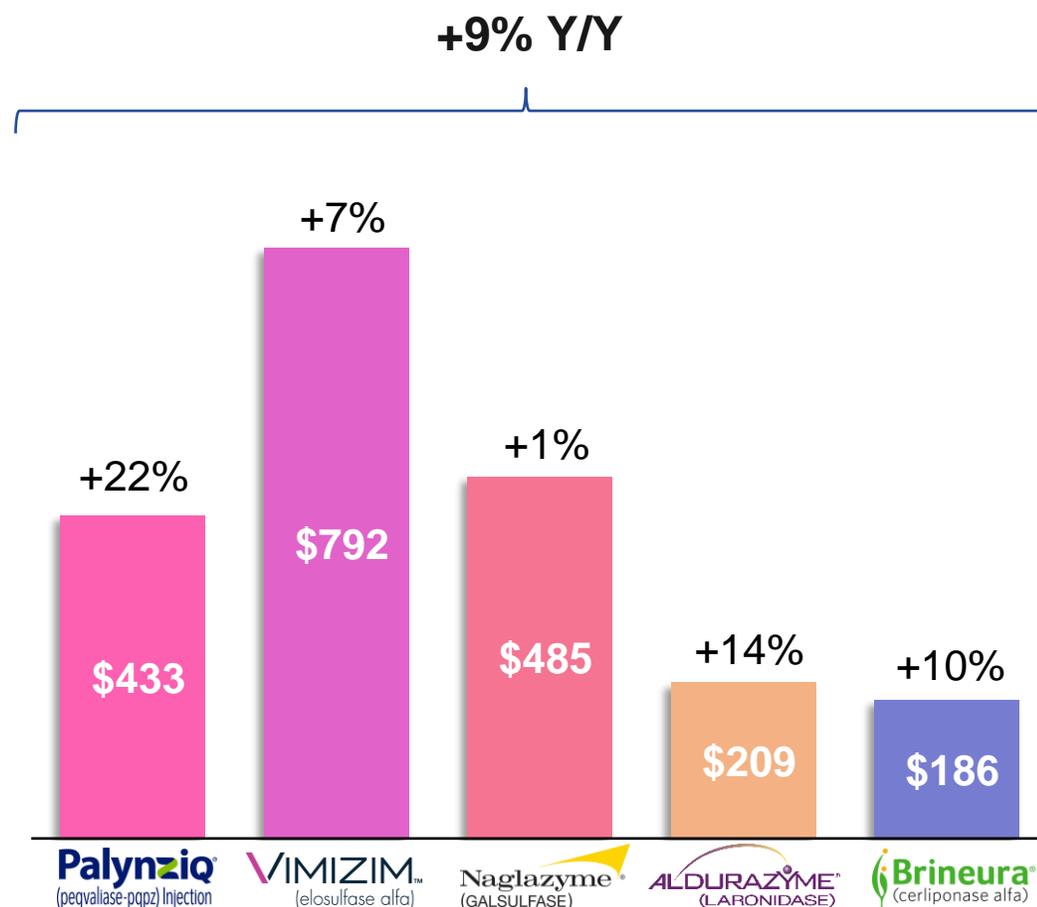


Cristin Hubbard
Chief Commercial Officer

Strong Enzyme Therapies Growth Led by PALYNZIQ

Enzyme Therapies FY'25 Revenue

\$ in millions



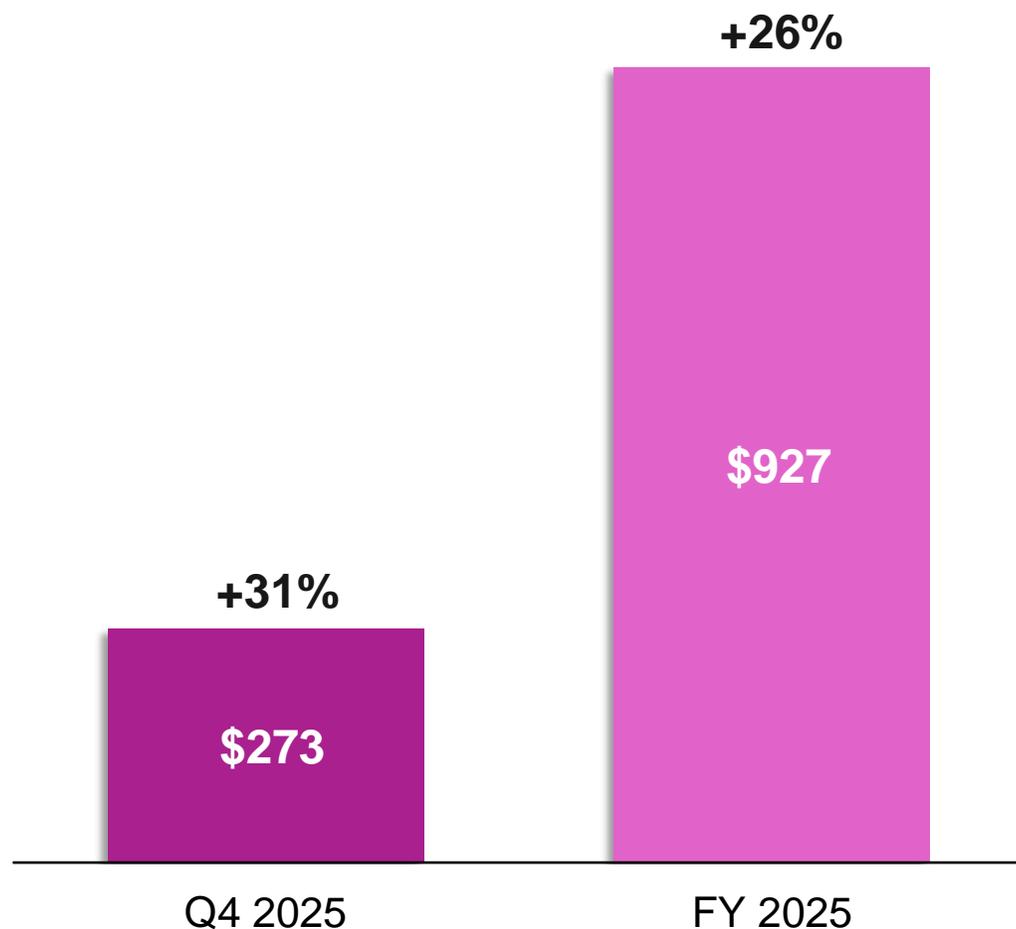
Growth across every product in Enzyme Therapies portfolio, Y/Y

- **9% Y/Y** growth driven by new patient starts across all products and consistently high adherence rates
- **Potential PALYNZIQ Label Expansion in 2026:**
 - Only therapy to enable physiologic Phe levels while reducing dietary restrictions, regardless of severity
 - **February 28, 2026:** U.S. PDUFA target action date for anticipated **adolescent label** expansion
 - Caregiver support during titration period with PALYNZIQ may drive adherence during adulthood
 - Potential for PALYNZIQ-treated adolescents to benefit from Phe lowering and reduced need for dietary therapy

VOXZOGO: FY'25 ↑26% Y/Y Driven by Global Expansion

VOXZOGO® Revenue

\$ in millions



Over 5,000 children with achondroplasia now treated with VOXZOGO; Global expansion expected in 2026

- Rapid uptake in newly launched markets in 2025
- In Q4'25, **approximately half** of new U.S. starts in under-2-year age group
- 2026 focused, multi-pronged global expansion:
 - Driving new starts across all ages worldwide, with emphasis on under-2-year age group
 - Continue to find and start treatment-eligible infants in highly penetrated incident markets
 - More deeply penetrate larger countries
 - Expand uptake in fast-growing, newly launched markets
- sNDA submission for full approval of VOXZOGO expected **Q2'26**, leveraging extensive safety and efficacy data package

Research & Development Update



Greg Friberg
Chief R&D Officer

Evidence that Cumulative Growth with VOXZOGO Treatment from Infancy Provides Clinically Important Health Benefits

Preventing complications

Neurological

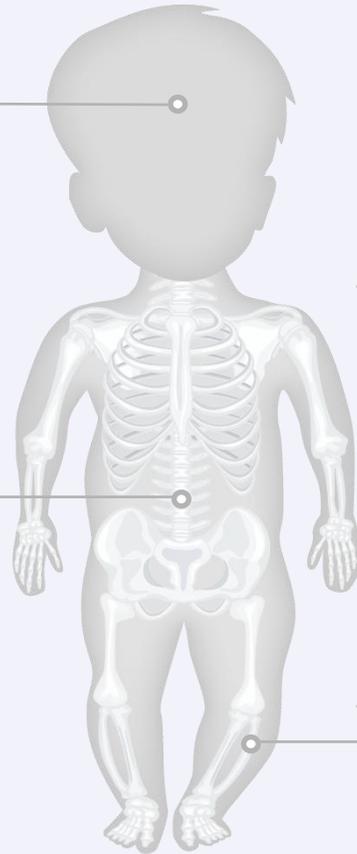
Foramen magnum stenosis & sleep testing

- In a retrospective study, no children who initiated vosoritide aged <2 years developed foramen magnum stenosis and vosoritide initiation in children aged <3 years improved sleep quality¹

Spinal

Symptomatic spinal stenosis

- In a prospective observational study, vosoritide improved spinal alignment in young children with ACH after 1 year of treatment²



Musculoskeletal

Body proportionality

- In clinical trials, vosoritide improved upper-to-lower body segment ratio vs untreated children^{3,4}

Genu varum

- In a prospective observational study, vosoritide improved genu varum in young children with ACH after 1 year of treatment²

Increased Functionality

Improved mobility/gait

- In an observational study, vosoritide provided clinically significant improvements in the 6-minute walking distance test from baseline after 1 year of treatment⁵

Improved QOL

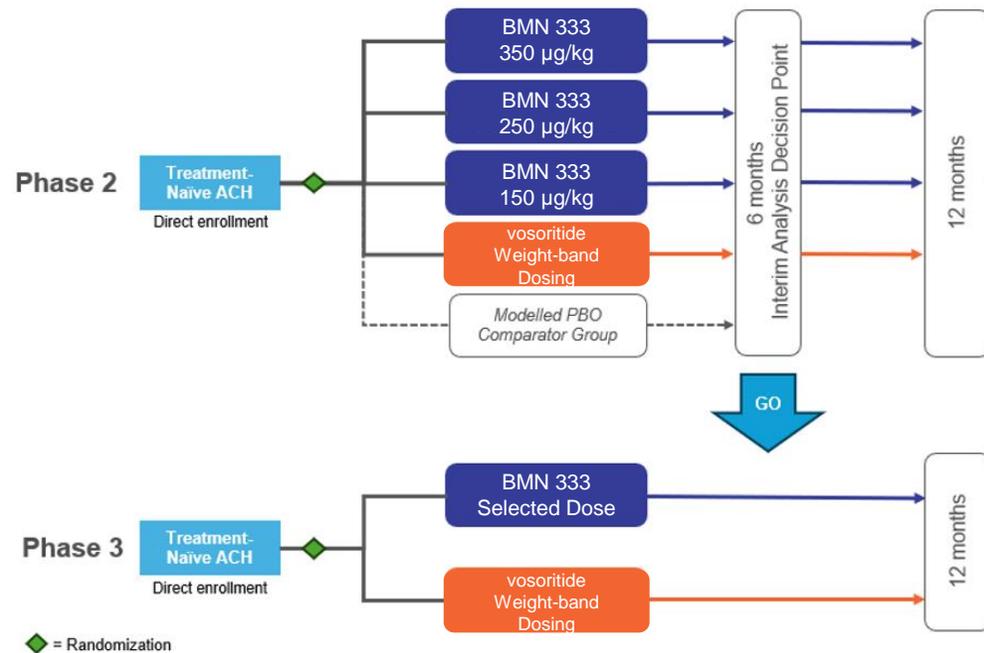
- In a clinical trial, vosoritide positively impacted physical and social QOL after early treatment initiation in children with ACH after 3 years⁶

As presented
at ESPE/ESE 2025

BMN 333: Phase 2/3 Study Design for BioMarin's Long-Acting CNP

Registration-enabling Study Designed to Target Superior Profile; Planned for 1H'26 Start

BMN 333 Phase 2/3 Study Design¹ Enrollment to Begin 1H'26



Key Highlights

- Phase 2 dose-ranging portion planned to include **three dose levels of BMN 333 vs. VOXZOGO**
- Phase 3 design:
 - Comparison of BMN 333 weekly against active control (VOXZOGO): 60 patients in each arm
 - **90%** power to detect a **50%** increase in annualized growth velocity (AGV) vs. VOXZOGO; infers a minimal detectable increase of **2.25 cm/yr** over placebo
- Will assess additional measures of health and wellness, including proportionality and quality of life
- Goal is to establish BMN 333 as new **standard of care** for achondroplasia

¹Study design subject to change post-feedback from regulatory authorities

Anticipated 2026 Pipeline Highlights Across Therapeutic Areas

3 Major Data Readouts and Planned Submissions for Regulatory Approval

VOXZOGO
(vosoritide) for injection

hypochoondroplasia
(Phase 3 data)

VOXZOGO
(vosoritide) for injection

achondroplasia
Full Approval

BMN 401

ENPP1 deficiency
(Phase 3 data)

6 New Opportunities across Skeletal Conditions

VOXZOGO
(vosoritide) for injection

5 new indications:
hypochoondroplasia, idiopathic short stature, Noonan syndrome, Turner syndrome, SHOX deficiency

BMN 333

Long-acting CNP

New product for achondroplasia

2 Label Expansions Expected for Adolescents¹

Palynziq
(pegvaliase-pqpz) Injection
PKU

Pombiliti + **Opfolda**
(cipaglucosidase alfa-atga) (miglustat) 65 mg capsules
Pompe disease

2 Program Updates to Enable Registration-enabling Studies

BMN 351
Duchenne muscular dystrophy
(Phase 1/2 data)

BMN 333
Long-acting CNP for achondroplasia
(Phase 1 data)

¹Pending closure of BioMarin's acquisition of Amicus (including Pombiliti + Opfolda); acquisition is expected to close in Q2'26, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions

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 December 31,		Twelve Months Ended December 31,	
	2025	2024	2025	2024
GAAP Reported Net Income (Loss)	\$ (47)	\$ 125	\$ 349	\$ 427
Adjustments				
Stock-based compensation expense - COS	4	3	14	15
Stock-based compensation expense - R&D	14	14	55	60
Stock-based compensation expense - SG&A	29	34	113	127
Amortization of intangible assets	5	10	19	43
Acquisition-related costs ⁽²⁾	—	—	15	—
Gain on sale of nonfinancial assets ⁽³⁾	—	—	—	(10)
Severance and restructuring costs ⁽⁴⁾	124	10	124	96
Loss on investments ⁽⁵⁾	—	—	3	5
Income tax effect of adjustments	(40)	(16)	(78)	(76)
Non-GAAP Income	\$ 89	\$ 180	\$ 614	\$ 686

Reconciliation of GAAP and Non-GAAP R&D and SG&A Expenses⁽¹⁾

	Three Months Ended December 31,				Twelve Months Ended December 31,			
	2025		2024		2025		2024	
	R&D	SG&A	R&D	SG&A	R&D	SG&A	R&D	SG&A
GAAP expenses	\$ 192	\$ 446	\$ 174	\$ 267	\$ 922	\$ 1,153	\$ 747	\$ 1,009
Adjustments								
Stock-based compensation expense	(14)	(29)	(14)	(34)	(55)	(113)	(60)	(127)
Acquisition-related costs ⁽²⁾	—	—	—	—	—	(15)	—	—
Severance and restructuring costs ⁽⁴⁾	—	(124)	—	(10)	—	(124)	—	(96)
Non-GAAP expenses	\$ 178	\$ 292	\$ 159	\$ 222	\$ 867	\$ 901	\$ 688	\$ 786

Reconciliation of GAAP Income from Operations to Non-GAAP Income from Operations⁽¹⁾

	Three Months Ended December 31,				Twelve Months Ended December 31,			
	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 (Loss) from Operations	\$ (45)	(5.1)%	\$ 161	21.6 %	\$ 409	12.7 %	\$ 484	17.0 %
Adjustments								
Stock-based compensation expense	47	5.4	51	6.8	182	5.7	202	7.1
Amortization of intangible assets	5	0.6	10	1.3	19	0.6	43	1.5
Acquisition-related costs ⁽²⁾	—	—	—	—	15	0.5	—	—
Gain on sale of nonfinancial assets ⁽³⁾	—	—	—	—	—	—	(10)	(0.4)
Severance and restructuring costs ⁽⁴⁾	124	14.2	10	1.3	124	3.8	96	3.4
Non-GAAP Income from Operations	\$ 132	15.1 %	\$ 232	31.1 %	\$ 750	23.3 %	\$ 815	28.6 %

Reconciliation of GAAP Diluted EPS to Non-GAAP Diluted EPS⁽¹⁾

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2025	2024	2025	2024
GAAP Diluted EPS	\$ (0.24)	\$ 0.64	\$ 1.80	\$ 2.21
Adjustments				
Stock-based compensation expense	\$ 0.24	\$ 0.26	\$ 0.92	\$ 1.03
Amortization of intangible assets	\$ 0.03	\$ 0.05	\$ 0.10	\$ 0.22
Acquisition-related costs ⁽²⁾	\$ —	\$ —	\$ 0.08	\$ —
Gain on sale of nonfinancial assets ⁽³⁾	\$ —	\$ —	\$ —	\$ (0.05)
Severance and restructuring costs ⁽⁴⁾	\$ 0.63	\$ 0.05	\$ 0.63	\$ 0.49
Loss on investments ⁽⁵⁾	\$ —	\$ —	\$ 0.02	\$ 0.03
Income tax effect of adjustments	\$ (0.20)	\$ (0.08)	\$ (0.40)	\$ (0.39)
Non-GAAP Diluted EPS	<u>\$ 0.46</u>	<u>\$ 0.92</u>	<u>\$ 3.15</u>	<u>\$ 3.52</u>

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

(2) These amounts were included in SG&A and represent severance costs incurred in the acquisition of Inozyme in July 2025.

(3) Represents a payment triggered by a third party's attainment of a regulatory approval milestone related to previously sold intangible assets.

(4) These amounts were included in SG&A and represent impairment of long-lived assets, severance and other restructuring costs related to the company's 2025 strategic decision to voluntarily withdraw ROCTAVIAN from the market and 2024 corporate initiatives and the associated organizational redesign efforts.

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