First Quarter 2026 Earnings

May 4, 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 Diluted EPS and operating cash flow for, in certain instances, the full-year 2026, second quarter and second half of 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, including PALYNZIQ, and VOXZOGO, and the expected impact of the acquisition of Amicus Therapeutics, Inc. (Amicus); the anticipated benefits of the acquisition of Amicus, including the addition of GALAFOLD and POMBILITI + OPFOLDA to BioMarin's portfolio; 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 May 4, 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 Annual Report on Form 10-K for the year ended December 31, 2026, 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 17 for the reconciliations to the comparable information reported under U.S. GAAP.

Q1'26 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

Q1'26 Key Business Updates

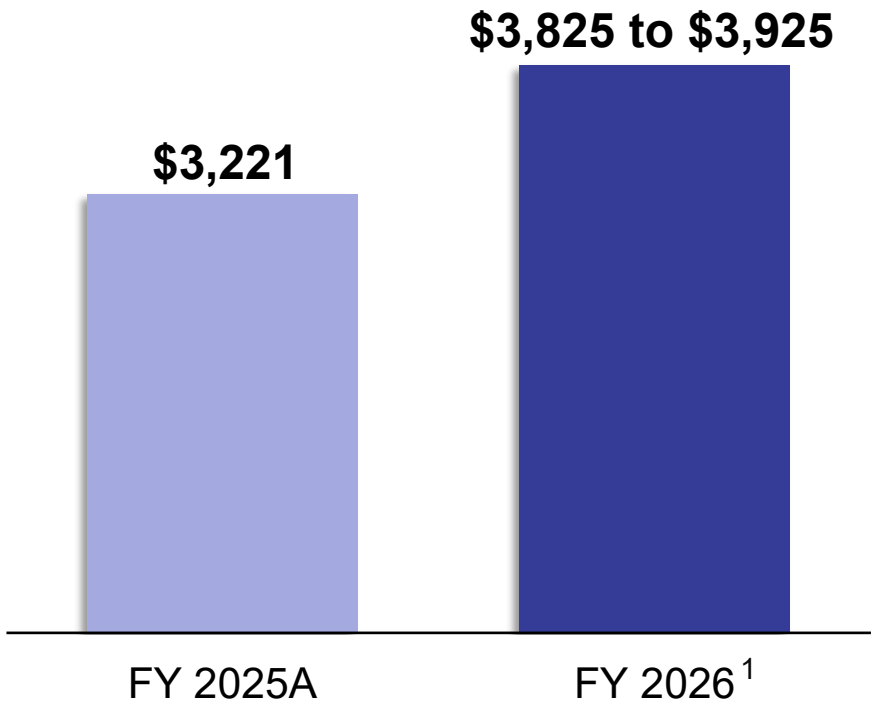
Alexander Hardy
Chief Executive Officer

Poised for Accelerated Growth in 2026

Updated FY'26 Total Revenues Guidance

$ in millions

+20%[1] Y/Y



Anticipated Key Highlights in 2026

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® (elosulfase alfa)
Brineura® (cerliponase alfa)
ALDURAZYME® (LARONIDASE)
Galafold® (migalastat)
Pombiliti™ + Opfolda™ (cipaglucosidase alfa-atga) (miglustat) 65 mg capsules
VOXZOGO® (vosoritide) for injection

[1]Midpoint of updated FY 2026 Total Revenues guidance

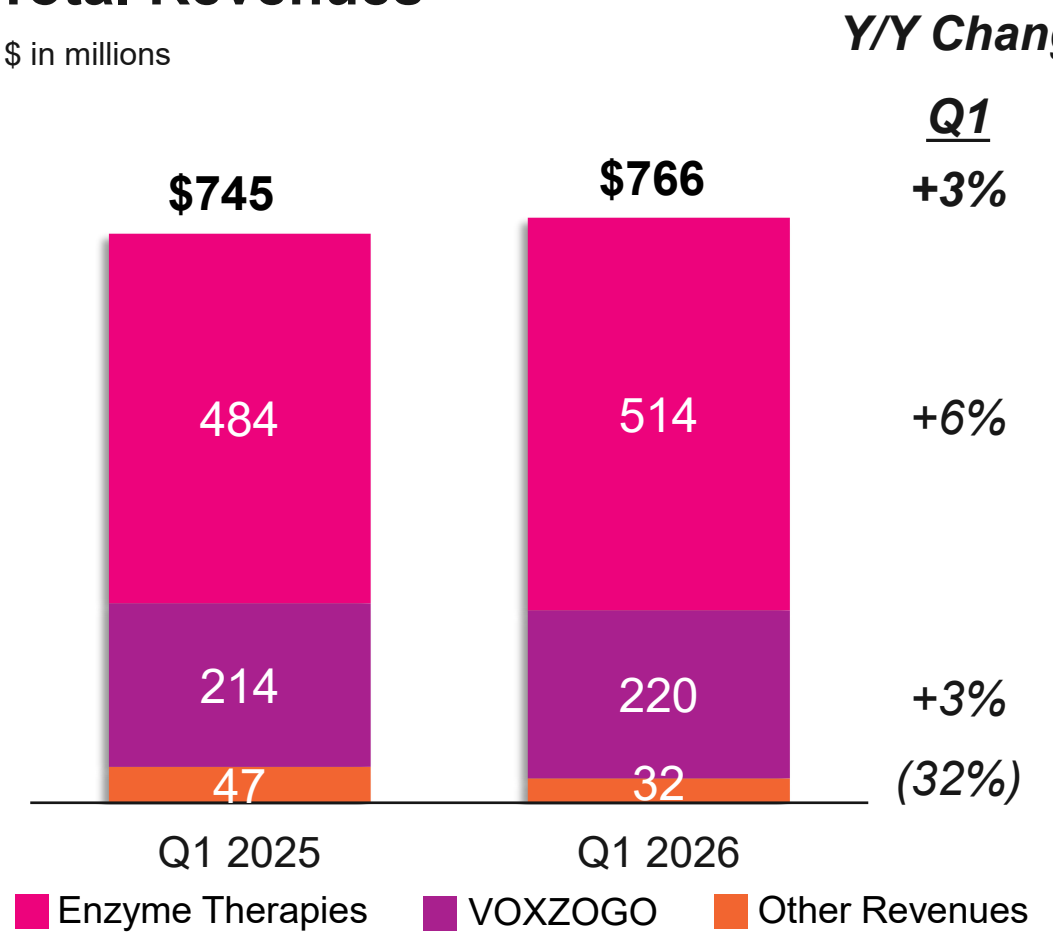
Q1'26 Financial Results

Brian Mueller
Chief Financial Officer

Solid Q1’26 Revenue; 2H’26 Outlook Ramps Significantly

Total Revenues

$ in millions



Enzyme Therapies

- Q1’26 revenue increased **6%**, Y/Y, led by VIMIZIM, NAGLAZYME, and BRINEURA
- PALYNZIQ revenue reflected elevated U.S. stocking in Q4’25; New patient starts grew in Q1
- Anticipate Y/Y revenue growth for PALYNZIQ in full-year 2026

VOXZOGO

- Revenue increased **3%** Y/Y
- New patient starts across all regions
- Y/Y results reflect the timing of large international orders in Q4’25, as anticipated
- Consistent with 2025, revenue expected to be weighted toward 2H’26

Enzyme Therapies include ALDURAZYME, BRINEURA, KUVAN, NAGLAZYME, PALYNZIQ, VIMIZIM

Q1'26 Results Reflect Impact of Cost of Sales and Pre-Close Charges

Q1 2026 Results

In millions, except percentages and per share amounts	Q1'26	Y/Y Change
GAAP Cost of Sales	$195	28%
GAAP R&D	$179	+13%
GAAP SG&A	$258	+25%
GAAP Operating Margin	16.9%	(13.1) ppts
Non-GAAP R&D[1]	$167	+14%
Non-GAAP SG&A[1]	$222	+21%
Non-GAAP Operating Margin[1]	24.3%	(11.4) ppts
GAAP Diluted EPS	$0.54	(43%)
Non-GAAP Diluted EPS[1]	$0.76	(33%)
Operating Cash Flow	$221	27%

Cost of Sales

- NAGLAZYME charge of ~$31M expected to be offset in FY'26 Non-GAAP Diluted EPS guidance

Non-GAAP R&D and SG&A

- R&D Y/Y increase primarily driven by BMN 401
- SG&A Y/Y increase driven by commercial expansion across Enzyme Therapies and VOXZOGO, and Amicus pre-close costs

Non-GAAP Diluted EPS

- **$0.76** EPS impacted by NAGLAZYME charge and Amicus pre-close costs totaling ~$0.20

Operating Cash Flow

- Generated operating cash flow of **$221M** in Q1 to support continued investment in innovation

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

Updated Full-year 2026 Guidance

(Including Post-close Contributions from Amicus Acquisition[1])

(In millions, except per share amounts)	Prior Guidance As of February 23, 2026	Updated Guidance As of May 4, 2026
Total Revenues	$3,325 to $3,425	$3,825 to $3,925
Enzyme Therapies	$2,225 to $2,275	$2,725 to $2,775
VOXZOGO	$975 to $1,025	Unchanged
Other Revenues	$100 to $125	Unchanged
Non-GAAP Diluted EPS[2,3,4]	$4.95 to $5.15	$4.85 to $5.05

2026 Anticipated Quarterly Dynamics

- More than 55% of 2026 Total Revenues expected in 2H'26
- Q2 Non-GAAP Diluted EPS expected to be modestly higher vs. Q1 EPS
- ~Two-thirds of FY'26 Non-GAAP Diluted EPS expected in 2H'26

Key Considerations

- Updated guidance reflects post-close contributions from Amicus beginning April 27, 2026.
- As previously communicated, the acquisition of Amicus is expected to be slightly dilutive to full-year 2026 Non-GAAP Diluted EPS; historical BioMarin Non-GAAP Diluted EPS guidance is unchanged.
- The Amicus acquisition will be accounted for as a business combination, which will result in intangible amortization impacting GAAP results over future periods and excluded from Non-GAAP results. BioMarin will continue to include interest expense related to the Amicus financing in both GAAP and Non-GAAP financial results. Guidance is subject to change based on various factors including finalization of purchase accounting.

[1]Post-close contributions refer to revenue, operating expense and incremental interest expense associated with acquisition financing after closing of the Amicus transaction on April 27, 2026; [2]Refer to slide 2 for more detail on Non-GAAP financial measures; [3]Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding; [4]Non-GAAP Diluted EPS guidance assumes a combined company tax rate of 22%, which is subject to change as the company completes its integration activities and purchase accounting.

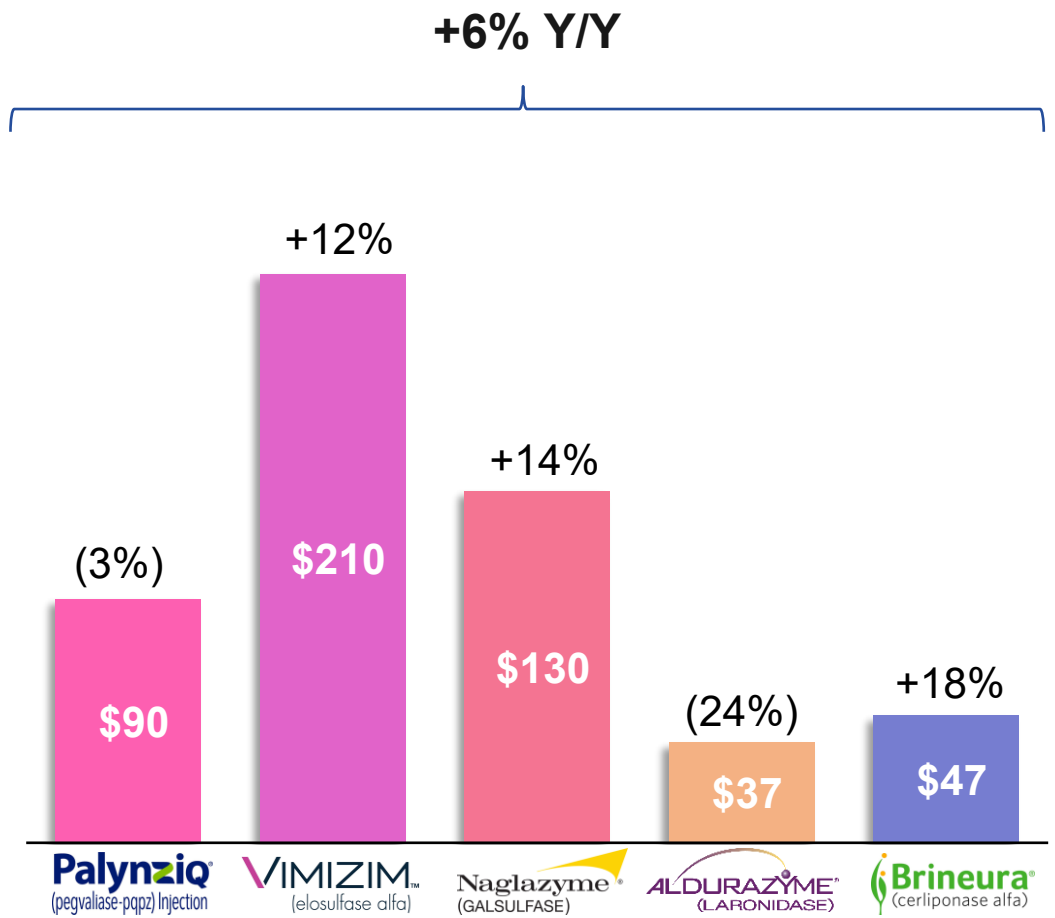
Commercial Update

Cristin Hubbard
Chief Commercial Officer

Enzyme Therapies: Durable Performance in Q1'26

Enzyme Therapies Q1'26 Revenue

$ in millions



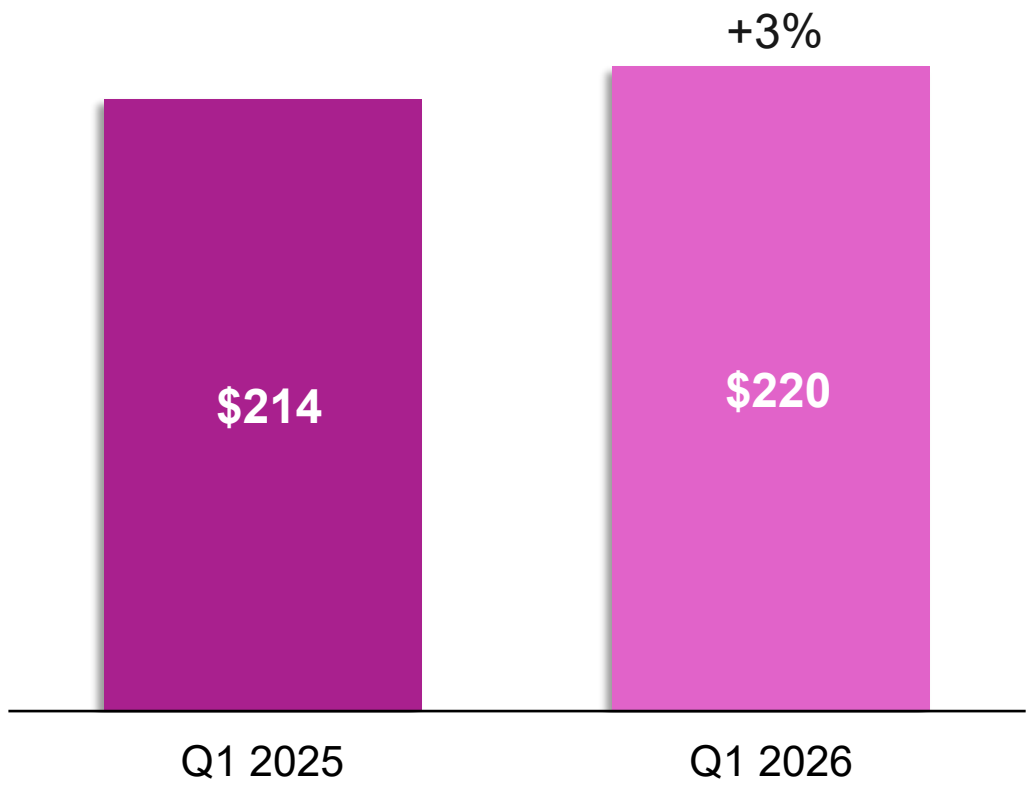
Strong Patient Demand; Early Momentum in PALYNZIQ Adolescent Launch

- **6%** Y/Y growth, led by VIMIZIM, NAGLAZYME, and BRINEURA
- PALYNZIQ Key Dynamics:
  - Continued expansion of underlying patient base
  - Adolescent (12+) U.S. launch initiated, with strong early momentum; meaningful enrollments and new starts from <18y/o
  - Adolescent uptake driven by experienced PALYNZIQ prescribers with newer prescribers gaining momentum
  - PALYNZIQ remains the only therapy enabling physiologic Phe control with reduced dietary restrictions
  - Adolescents EU approval anticipated in 2026

Skeletal Conditions: Strong Underlying Demand in Q1'26

VOXZOGO® Revenue

$ in millions



Q1'26 Dynamics

- Ongoing patient additions across all regions and strong adherence; ~75% Q1 VOXZOGO revenue OUS[1]
- More than 20% Y/Y increase in children treated with VOXZOGO
- Focus on early diagnosis and treatment globally
  - > 50% Q1 new U.S. patient starts in < 2 y/o age cohort
  - Identifying patients in highly penetrated incident markets
- Deepening penetration across age groups in markets with significant growth potential

Pipeline Highlights

- Submitted sNDA for full approval in achondroplasia in April
- Advancing preparations for potential expansion into hypochondroplasia (HCH)
- Phase 3 topline data for HCH expected Q2'26; regulatory submissions planned 2H'26 with potential approval in 2027

Percentages reflect year-over-year change; [1]OUS = Outside of the United States

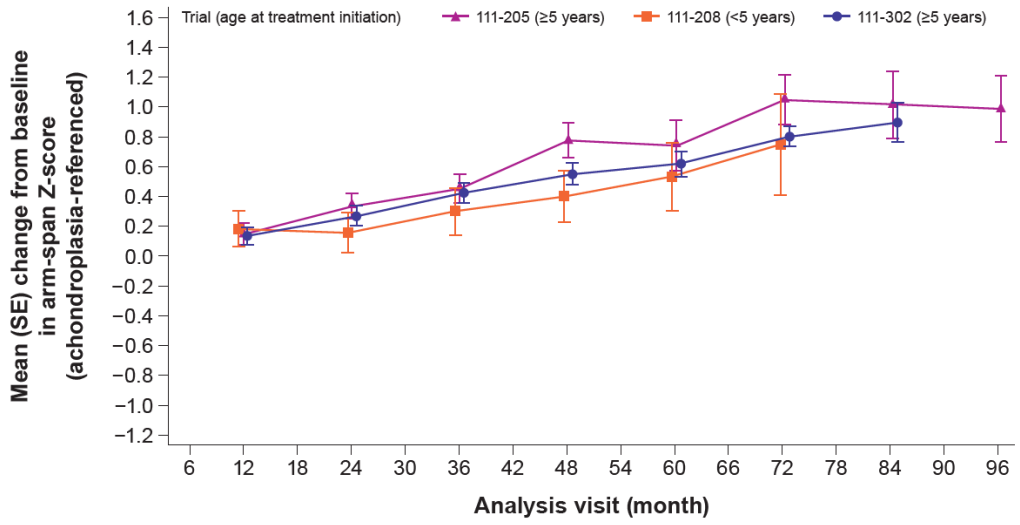
Research & Development Update

Greg Friberg
Chief R&D Officer

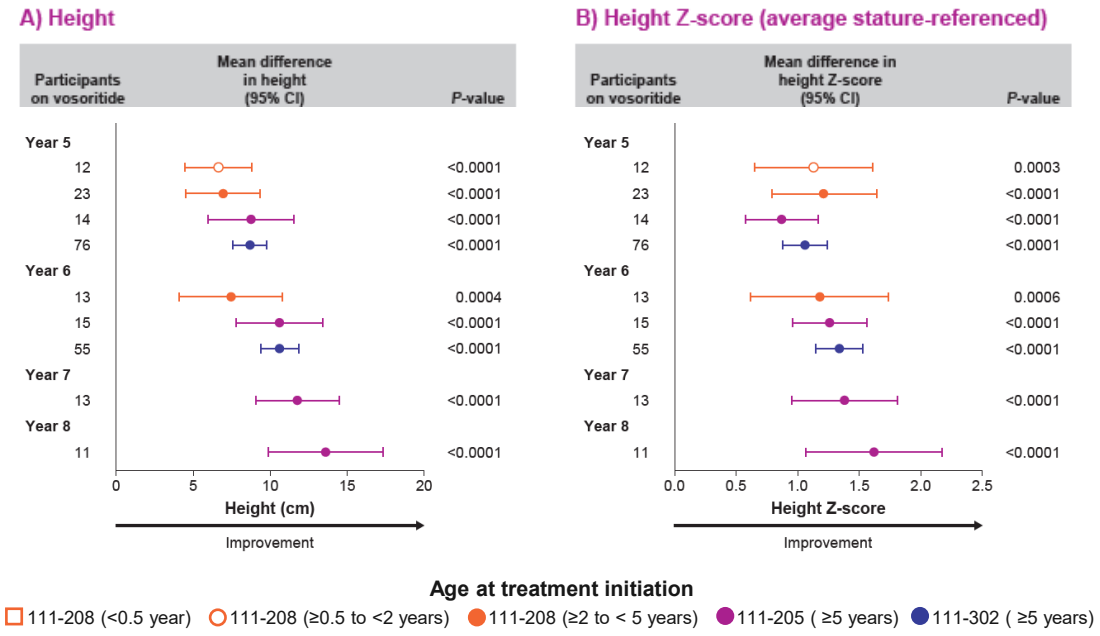
VOXZOGO: Expanding Evidence to Support Long-term Benefit

Data presented at the Pediatric Endocrine Society (PES) annual meeting showed sustained improvements across growth, proportionality, and quality-of-life related measures

Arm Span Z-score (ACH-referenced) Improved Over Time Across All Ages Independent of Age at Treatment Initiation



Height and Height Z-score (average stature-referenced) Improved in All Ages over Time (compared to ACH natural history)



Long-term data underpin the full approval submission and reinforce VOXZOGO's differentiated profile as the only therapy approved from infancy

Arm Span: Z-scores were referenced to age- and sex-matched untreated children with achondroplasia, SE, standard error.
Height: Baseline-adjusted cross-sectional analyses were performed using achondroplasia natural history (CLARITY)7 as a comparator. Results from 111-205 were rebaselined, with day 1 being the day of the first dose of 15 or 30 µg/kg vosoritide in study 111-202. CI, confidence interval.

2026 R&D Highlights

	Candidate	Condition	Key Highlights
Enzyme Therapies	Palynziq (pegvaliase-pqpz) Injection	Phenylketonuria	✓ Adolescents (12-17 y/o) U.S. Approval February • Adolescents (12-15 y/o) EU Approval Decision 2026
Skeletal Conditions	BMN 401	ENPP1 deficiency	• Phase 3 Topline Data Q2'26 • Phase 3 Full Data (Medical Congress) • Submissions for Regulatory Approval 2H'26
	VOXZOGO (vosoritide) for injection	Hypochondroplasia	• Phase 3 Topline Data Q2'26 • Phase 3 Full Data (Medical Congress) • Submissions for Regulatory Approval 2H'26
	VOXZOGO (vosoritide) for injection	Achondroplasia	✓ Submitted sNDA for Full Approval in April • Expect sNDA Acceptance by Q3'26
Pipeline	BMN 333	Achondroplasia	• Phase 1 PK Data (ENDO) ✓ Phase 2/3 Registration-enabling Study Enrolling
	BMN 351	Duchenne muscular dystrophy	✓ Phase 1/2 12 mg/kg Cohort Enrolling • 12 mg/kg Data Update by Year-end

Includes both completed / ongoing (✓) and anticipated R&D highlights in 2026 (non-exhaustive)

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 March 31,	
	2026	2025
GAAP Reported Net Income	**$ 106**	**$ 186**
Adjustments		
Stock-based compensation expense - COS	4	2
Stock-based compensation expense - R&D	12	12
Stock-based compensation expense - SG&A	28	23
Amortization of intangible assets	4	5
Severance costs [2]	8	—
Loss on investments [3]	—	3
Income tax effect of adjustments	(13)	(10)
Non-GAAP Income	$ 149	$ 221

Please refer to slide 21 for footnotes to the table

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

	Three Months Ended March 31,			
	2026		2025	
	R&D	SG&A	R&D	SG&A
GAAP expenses	**$ 179**	**$ 258**	**$ 159**	**$ 206**
Adjustments				
Stock-based compensation expense	(12)	(28)	(12)	(23)
Severance costs [2]	—	(8)	—	—
Non-GAAP expenses	$ 167	$ 222	$ 147	$ 183

Please refer to slide 21 for footnotes to the table

Reconciliation of GAAP Income from Operations to Non-GAAP Income from Operations[1]

	Three Months Ended March 31,			
	2026	Percent of GAAP Total	2025	Percent of GAAP Total
GAAP Income from Operations	**$ 130**	**16.9 %**	**$ 224**	**30.0 %**
Adjustments				
Stock-based compensation expense	44	5.7	37	5.0
Amortization of intangible assets	4	0.5	5	0.7
Severance costs [2]	8	1.0	—	—
Non-GAAP Income from Operations	$ 186	24.3 %	$ 266	35.7 %

Please refer to slide 21 for footnotes to the table

Reconciliation of GAAP Diluted EPS to Non-GAAP Diluted EPS[1]

	Three Months Ended March 31,	
	2026	2025
GAAP Diluted EPS	**$ 0.54**	**$ 0.95**
Adjustments		
Stock-based compensation expense	0.22	0.19
Amortization of intangible assets	0.02	0.03
Severance costs [2]	0.04	—
Loss on investments [3]	—	0.02
Income tax effect of adjustments	(0.07)	(0.05)
Non-GAAP Diluted EPS[4]	$ 0.76	$ 1.13

(1) Certain amounts may not sum or recalculate due to rounding.
(2) These amounts were included in SG&A and represent charges for severance in connection with the company's plan to simplify its organizational design and strategic initiatives in the first quarter of 2026.
(3) Represents impairment loss on non-marketable equity securities recorded in Other income (expense), net, in the first quarter of 2025.
(4) Both GAAP and Non-GAAP Weighted-Average Diluted Shares Outstanding were 197.7 million and 196.5 million shares for the three months ended March 31, 2026 and 2025, respectively.