

Third Quarter 2025 Earnings

October 27, 2025

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, Non-GAAP Diluted EPS and Operating Cash Flow for, in certain instances, the full-year 2025 and future periods, and the underlying drivers of those results, such as the revenue opportunity represented by treatments for Skeletal Conditions, namely VOXZOGO and VOXZOGO's expected fourth quarter 2025 performance and contribution to full-year 2025 Total Revenues, the expected demand and continued growth of BioMarin's Enzyme Therapies portfolio, and the anticipated benefits of BioMarin's acquisition of Inozyme Pharma, Inc.; the timing of orders for commercial products; BioMarin's ability to meet product demand; 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, data readouts, submissions, filings, and approvals; plans regarding ROCTAVIAN, including plans to pursue options to divest ROCTAVIAN, including exploring out-licensing opportunities; 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, 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 October 27, 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 June 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 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. 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 21 for the reconciliations to the comparable information reported under U.S. GAAP.

Q3'25 Earnings Agenda:

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



Alexander Hardy
President and CEO



Brian Mueller
Chief Financial Officer



Cristin Hubbard
Chief Commercial Officer



Greg Friberg
Chief R&D Officer

Q3'25 Key Business Updates

Alexander Hardy

President and Chief Executive Officer

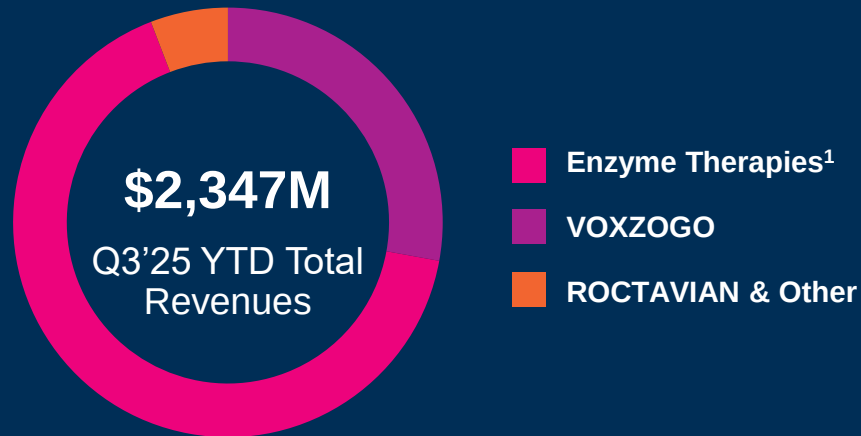


Building on Our Strong Foundation to Drive Growth in 2025 and Beyond

Year-to-date 2025 Total Revenues

↑ 11% Y/Y

Led by **more than 20% Y/Y** revenue growth for **PALYNZIQ** and **VOXZOGO**



Executing on Focused Strategy

- ✓ Raising Full-year 2025 Total Revenues guidance to between **\$3,150M** and **\$3,200M²**; **11% FY'25 growth Y/Y³**
- ✓ Reaffirming VOXZOGO FY'25 revenue outlook of between \$900M and \$935M
- ✓ Focused Portfolio Strategy; Pursuing Options to Divest ROCTAVIAN

Q3'25 Financial Results

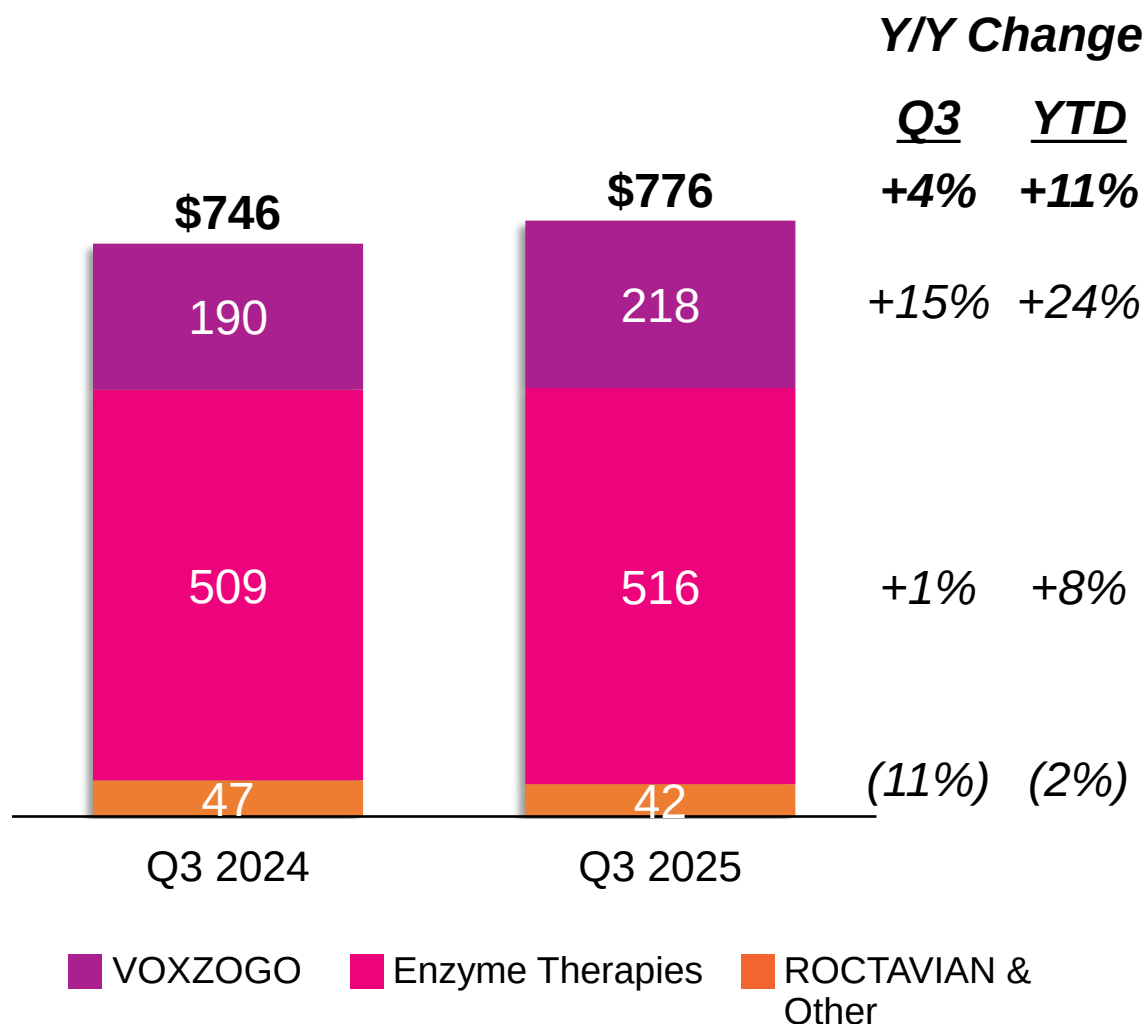
Brian Mueller

Executive Vice President, Chief Financial Officer



VOXZOGO & PALYNZIQ Strength Drive Increased FY'25 Guidance

Total Revenues (\$M)



FY'25 Total Revenues guidance increased to
\$3,150M – \$3,200M

VOXZOGO

- Strong global demand and new patient increases Q/Q
- Q3 year-to-date revenue increased **24%**, Y/Y
- Reaffirming **\$900M – \$935M** FY'25 revenue

Enzyme Therapies

- Q3 year-to-date revenue increased **8%**, Y/Y
- PALYNZIQ strength continues; **third consecutive quarter** of **20%+ Y/Y** revenue growth
- Q3'25 Enzyme Therapies revenue lower than Q2'25; strong underlying demand offset by large orders for NAGLAZYME and VIMIZIM in Q2
- Higher Q3'24 ALDURAZYME volume

Q3'25 Reflects Acquired IPR&D Impact; Strong Underlying Execution

Q3 2025 and YTD Results

In millions, except percentages	Q3'25	Q3 Y/Y	YTD Y/Y
GAAP R&D	\$409	+121%	+27%
GAAP SG&A	\$268	6%	(5%)
GAAP Operating Margin	(6.0%)	(21.3) ppts	+4.1 ppts
Non-GAAP R&D ¹	\$395	+127%	+30%
Non-GAAP SG&A ¹	\$223	+23%	+8%
Non-GAAP Operating Margin ¹	2.8%	(24.9) ppts	(1.4) ppts
GAAP Diluted EPS	(\$0.16)	(129%)	31%
Non-GAAP Diluted EPS ¹	\$0.12	(87%)	3%
Operating Cash Flow	\$369	+66%	+88%

Q3 Results Include Impact of \$221M Acquired IPR&D Charges on a Pre-Tax Basis

Non-GAAP Operating Margin and Non-GAAP Diluted EPS

- Q3 acquired IPR&D reduced Non-GAAP Operating Margin and Non-GAAP Diluted EPS (~\$1.10 per share impact)
- Year-to-date EPS growth reflects **underlying strong revenue performance and operational efficiencies**
- FY'25 Non-GAAP Operating Margin guidance updated to **26% – 27%** and FY'25 Non-GAAP Diluted EPS guidance updated to **\$3.50 – \$3.60**

Operating Cash Flow

- Significant operating cash flow growth continues, with **\$369M** generated in Q3, contributing to ~\$2B cash and investments balance as of quarter-end

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

Updated Full-year 2025 Guidance

<i>(In millions, except per share and % data)</i>	Prior Guidance	Updated Guidance
	As of August 4, 2025	As of October 27, 2025
Total Revenues¹	\$3,125 to \$3,200	\$3,150 to \$3,200
Non-GAAP Operating Margin %²	33% to 34%	26% to 27%
Non-GAAP Diluted EPS^{2,3}	\$4.40 to \$4.55	\$3.50 to \$3.60

Guidance updated October 27, 2025 includes acquired IPR&D charges through Q3 2025 of **\$221 million** on a pre-tax basis, resulting in an approximate impact of **7%** on Non-GAAP Operating Margin, or **\$1.10** on a per share basis

¹VOXZOGO contribution to full-year 2025 Total Revenues expected to be in the range of \$900 million to \$935 million. 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.

Q3'25 Commercial Update

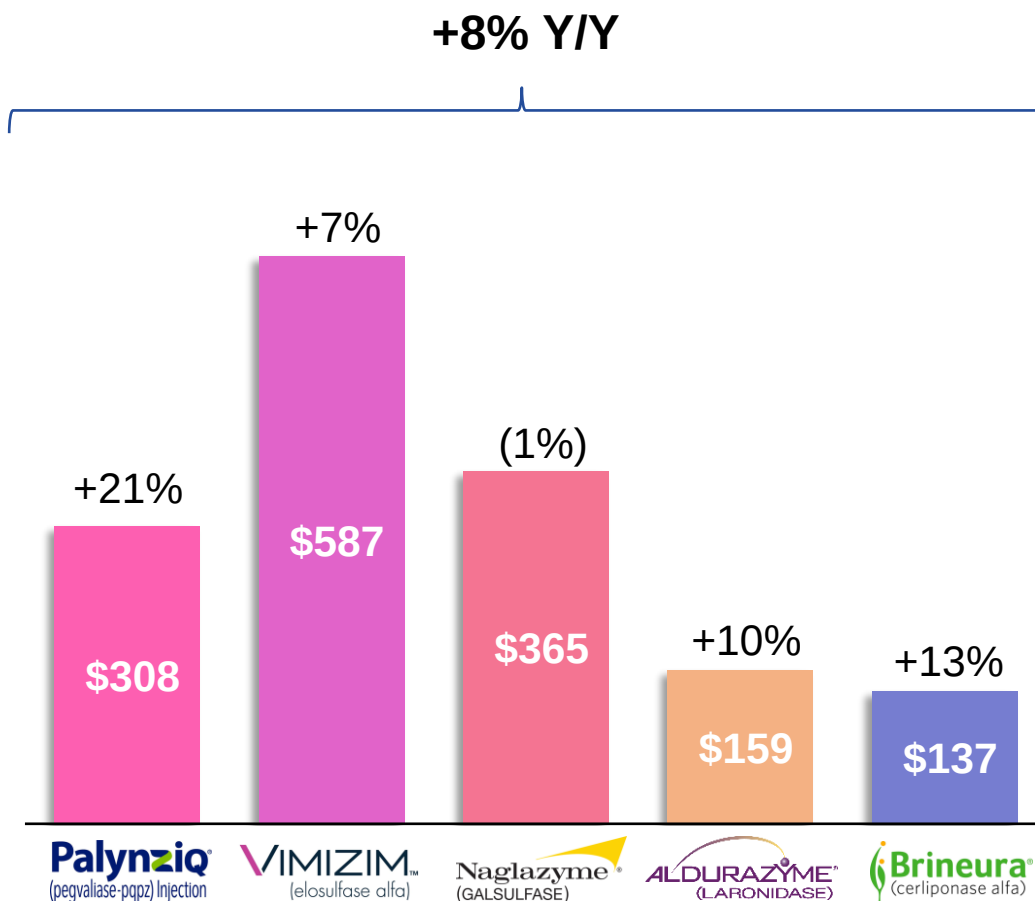
Cristin Hubbard

Executive Vice President, Chief Commercial Officer



Enzyme Therapies Growth Reflects Broad Uptake Across Portfolio

Enzyme Therapies Q3 YTD Revenue (\$M)

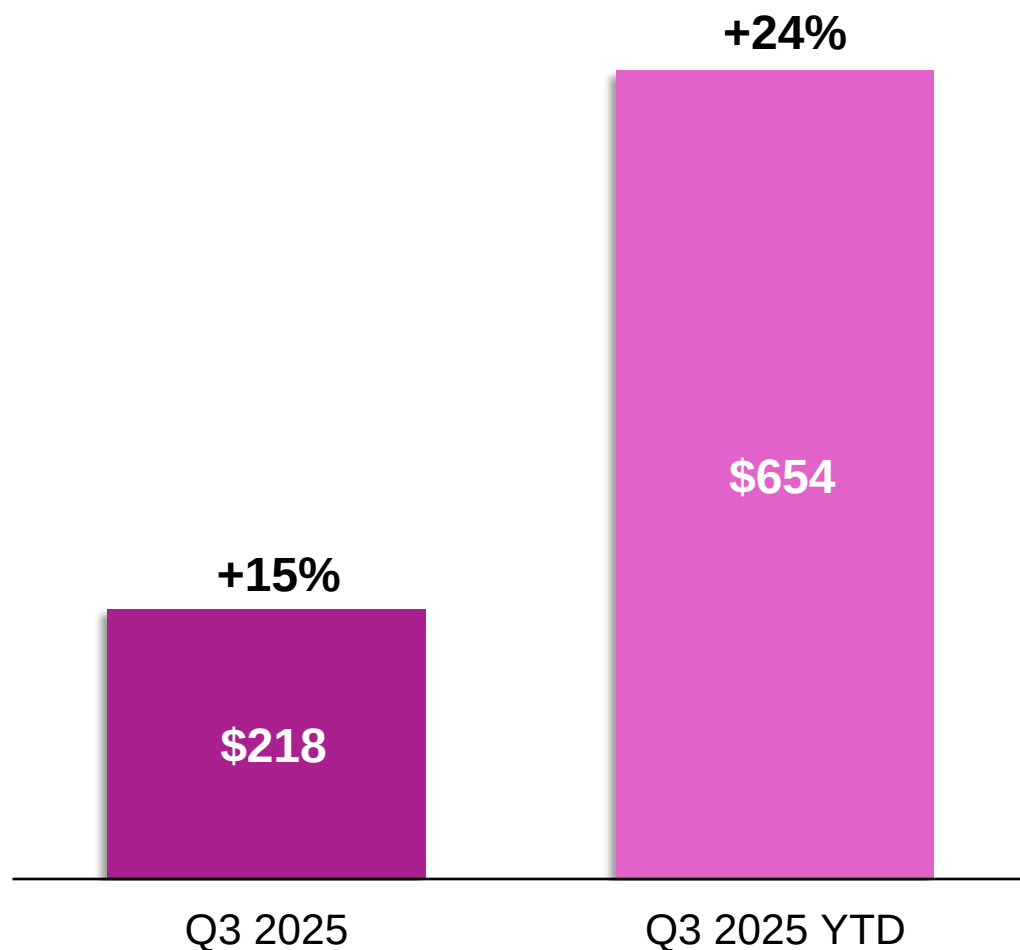


Year-to-date high-single-digit growth aligned with long-term outlook

- **Sustained PALYNZIQ growth** driven by greater numbers of patients titrating to daily maintenance dose and strong adherence
 - Pursuing age label expansion to adolescents in U.S. and Europe, with potential approval in **2026**
- YTD results reflect **increased new patient starts across all Enzyme Therapies**, despite quarter-to-quarter order timing dynamics

VOXZOGO: Q3 YTD ↑24% Y/Y; 2025 Outlook Reaffirmed

VOXZOGO® Revenue (\$M)



Q/Q new patient adds across all regions; Q4'25 VOXZOGO revenue expected to be highest of the year

- VOXZOGO available in **55** countries at end of Q3'25
- Continued new patient increases globally Q/Q
- ~**75%** year-to-date VOXZOGO revenue generated outside of the U.S. (OUS); strong uptake across international markets
- **U.S. dynamics in Q3'25**
 - Children under 2 years old represented of majority of Q3 U.S. new starts
 - Due to geographical dispersion of older children, initiatives were implemented to support uptake
 - VOXZOGO prescriber base continues to expand
- VOXZOGO Q4'25 revenue expected to be highest of the year driven by timing of large OUS orders, deeper penetration in existing markets and new patient starts

Building on our Leadership in Skeletal Conditions

Total Addressable Patient Population (TAPP): ~420,000

Focused on most severely impacted subset of children, representing modest proportion of TAPP

Indication	TAPP
Achondroplasia	24,000
Hypochondroplasia	14,000
Idiopathic short stature	190,000
Noonan syndrome	90,000
Turner syndrome	65,000
SHOX deficiency	40,000

Expansion Across Multiple New Indications

- Achondroplasia (ACH): **first of six** indications for treatment with VOXZOGO or BMN 333
- Preparing for VOXZOGO in hypochondroplasia (HCH) launch: pivotal data readout expected **1H'26**
- CANOPY Phase 2 clinical trials with VOXZOGO advancing across **four additional indications**: idiopathic short stature, Noonan syndrome, Turner syndrome, SHOX deficiency

VOXZOGO for HCH: Significant Breakthrough for Patients

Key Challenges to HCH Diagnosis



**Heterogenous
presentation**



**Complex referral
pathway**



**Low urgency to
diagnose**



**Various Barriers to
Genetic Testing**

Potential to Improve HCH Diagnosis

- **VOXZOGO in ACH** launch **experience** and commercial leadership has **laid the foundation** to scale and execute effectively in future indications
- **Global initiatives to improve early HCH diagnosis** include:
 - Targeted genetic reclassification
 - Clinician education
 - Patient/caregiver awareness
 - Diagnostic pathway optimization
- **Robust engagement and enthusiasm from HCPs** across specialties; reflection of the **growing recognition of the unmet needs** in HCH
- **VOXZOGO in HCH**: Phase 3 data expected **1H'26** and potential launch in **2027**

Q3'25 R&D Update

Greg Friberg

Executive Vice President, Chief R&D Officer

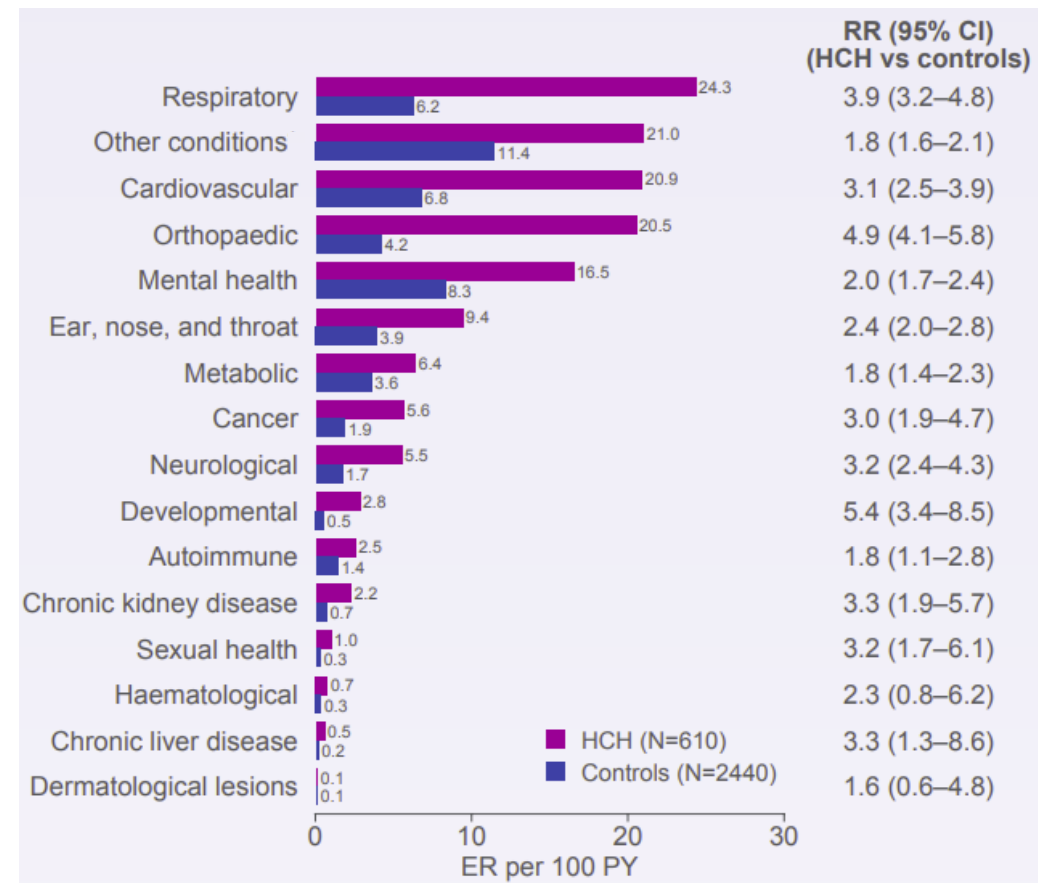


Potential to Address Significant Unmet Need in Hypochondroplasia

Hypochondroplasia Unmet Needs

- A retrospective study of children and adults with HCH showed **higher co-morbidities, procedures, and health care resource utilization** as compared to the general population¹
 - Prominent comorbidities included **respiratory, orthopedic, mental health, and ENT**
 - Overall, HCH patients had **higher rates of surgeries** driven by respiratory, orthopedic, and ENT
- **Quality of life is affected in individuals with HCH**, most notably due to impact on functional activities^{2,3}
 - Physical impacts of disproportionate short stature included difficulty performing daily tasks and reaching for objects
 - Social and emotional impacts included poor self-esteem, bullying, or difficulty fitting in with peers

Comorbidity Event Rates in HCH

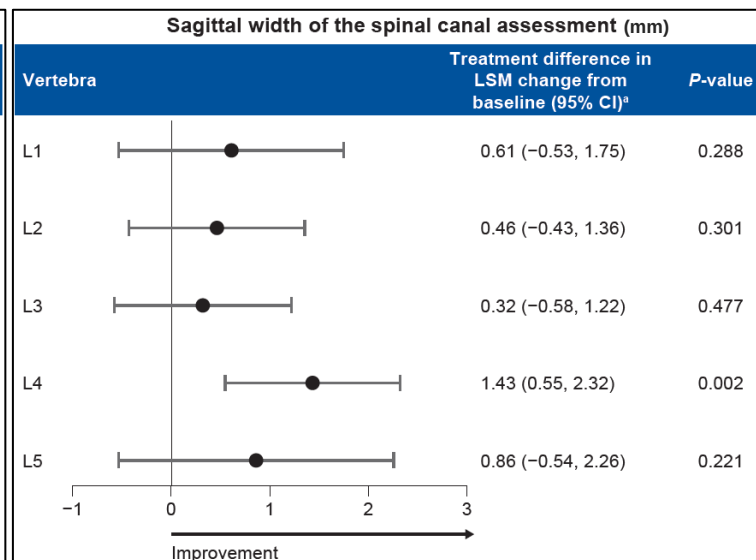
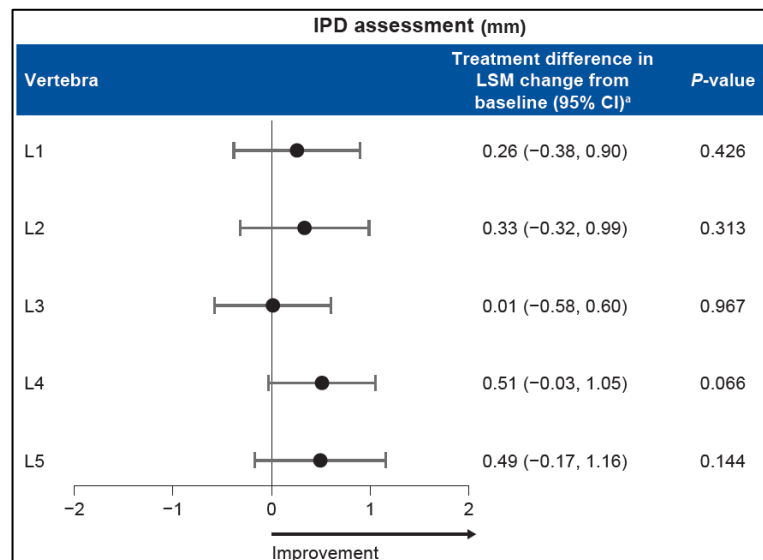


ER reported as the number of unique comorbidity episodes per 100 PY. Only the first occurring event within a 30-day period was counted to avoid over-counting events from the same episode
 Reddy et al., ICCBH 2024 (Poster P176)

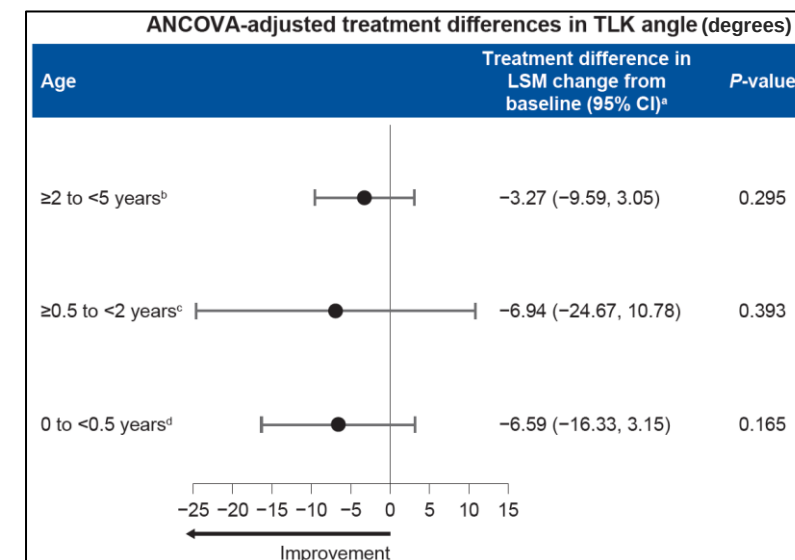
¹Mukherjee S et al. Medical Impact of HCH among children and adults in England: a 22 yr retrospective study (poster). ²Galetaki D et al. Horm Res Paediatr 2025; 17:1-9. ³Aldegheri R et al. J Pediatr Orthop B 2001; 10; 238-47

VOXZOGO Improves Spinal Morphology in Young Children with ACH, as Compared to Placebo Control

Positive effect of VOXZOGO on spinal measurements at week 52 compared with placebo



Positive effect of VOXZOGO on curvature of spine at week 52 compared with placebo



- VOXZOGO is the **only approved therapy** with data showing a **positive impact on spinal morphology**
- Spinal morphology is one of the factors that can lead to spinal stenosis, a leading cause of morbidity in achondroplasia

The LSM differences are reported in millimeters for the VOXZOGO vs placebo ANCOVA-adjusted treatment groups from baseline to week 52. ^aTreatment difference in LSM change was calculated as VOXZOGO - placebo. ANCOVA, analysis of covariance; CI, confidence interval; IPD, interpedicular distance; L1-L5, lumbar vertebrae 1-5; LSM, least squares mean.

Age cohorts represent age at treatment initiation. The LSM differences are reported in degrees for the VOXZOGO vs placebo ANCOVA-adjusted treatment groups from baseline to week 52. ^aTreatment difference in LSM change was calculated as VOXZOGO - placebo.

^bVOXZOGO, n = 11; placebo, n = 7. ^cVOXZOGO, n = 10; placebo, n = 7. ^dVOXZOGO, n = 18; placebo, n = 13. TLK; thoracolumbar kyphosis.

BMN 333: Phase 2/3 Designed to Target Superior Profile

BMN 333 for achondroplasia: Targeting initiation of **registration-enabling Ph 2/3** study, expected to begin **1H'26**

Biological Rationale for Potential Clinical Benefit

- **Preclinical data:** Suggest free CNP levels reached by BMN 333 may drive additional growth
- **Human genetics:** Pathway overexpression can drive significant growth without unexpected safety challenges
- **Clinical data:** Long-acting agents have not explored these higher exposures (BMN 333 aims to extend the dose-response curve)

Phase 2/3 Registration-enabling Study

- Four-arm, Phase 2 dose-ranging study planned to include three dose levels of BMN 333 vs. VOXZOGO
- Followed by Phase 3 comparison of BMN 333 against active control (VOXZOGO), assessing safety, growth, and functional outcomes

BMN 333 Designed for Superiority vs. VOXZOGO

- Aligned with regulators to pursue **superiority** for BMN 333
- Potential to set a **new standard of care** for the treatment of achondroplasia

Anticipated Key Pipeline Highlights over Coming Quarters

	2H 2025	1H 2026	2H 2026
Skeletal Conditions	 BMN 333 Ph1 PK data	BMN 333 Ph1 PK presentation	
Enzyme Therapies		BMN 333 Ph2/3 registration-enabling study initiation	
		VOXZOGO for HCH Ph3 data readout	VOXZOGO for HCH health authority submissions for approval
Pipeline	PALYNZIQ adolescents (12-17 y/o) U.S. and EU submissions	PALYNZIQ adolescents U.S. and EU launch	
		BMN 401 ENERGY 3 (1-12 y/o) data readout	BMN 401 ENERGY 3 U.S. and EU submissions
	BMN 351 for DMD ¹ Clinical update (6 & 9 mg/kg cohorts)	BMN 349 for AATD ² Phase 2 (POC) study initiation	

¹DMD = Duchenne muscular dystrophy; ²AATD = 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP Reported Net Income (Loss)	\$ (31)	\$ 106	\$ 395	\$ 302
Adjustments				
Stock-based compensation expense - COS	4	5	11	12
Stock-based compensation expense - R&D	15	11	41	45
Stock-based compensation expense - SG&A	30	27	83	93
Amortization of intangible assets	5	5	15	34
Acquisition-related costs ⁽²⁾	15	—	15	—
Gain on sale of nonfinancial assets ⁽³⁾	—	—	—	(10)
Severance and restructuring costs ⁽⁴⁾	—	44	—	86
Loss on investments ⁽⁵⁾	—	—	3	5
Income tax effect of adjustments	(16)	(20)	(38)	(61)
Non-GAAP Income	\$ 22	\$ 178	\$ 525	\$ 506

(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 severance and restructuring costs related to the Company's 2024 corporate initiatives and the associated organizational redesign efforts.

(5) 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 September 30,				Nine Months Ended September 30,			
	2025		2024		2025		2024	
	R&D	SG&A	R&D	SG&A	R&D	SG&A	R&D	SG&A
GAAP expenses	\$ 409	\$ 268	\$ 185	\$ 253	\$ 730	\$ 707	\$ 574	\$ 742
Adjustments								
Stock-based compensation expense	(15)	(30)	(11)	(27)	(41)	(83)	(45)	(93)
Acquisition-related costs ⁽²⁾	—	(15)	—	—	—	(15)	—	—
Severance and restructuring costs ⁽⁴⁾	—	—	—	(45)	—	—	—	(87)
Non-GAAP expenses	<u>\$ 395</u>	<u>\$ 223</u>	<u>\$ 173</u>	<u>\$ 182</u>	<u>\$ 689</u>	<u>\$ 609</u>	<u>\$ 529</u>	<u>\$ 563</u>

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(4) 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 September 30,				Nine Months Ended September 30,							
	Percent of GAAP Total Revenue		Percent of GAAP Total Revenue		Percent of GAAP Total Revenue		Percent of GAAP Total Revenue					
	2025		2024		2025		2024					
GAAP Income from Operations	\$	(47)	(6.0)%	\$	114	15.3 %	\$	454	19.4 %	\$	323	15.3 %
Adjustments												
Stock-based compensation expense		49	6.3		43	5.7		135	5.8		150	7.2
Amortization of intangible assets		5	0.6		5	0.7		15	0.6		34	1.6
Acquisition-related costs ⁽²⁾		15	1.9		—	— %		15	0.6		—	—
Gain on sale of nonfinancial assets ⁽³⁾		—	—		—	— %		—	—		(10)	(0.5)
Severance and restructuring costs ⁽⁴⁾		—	—		45	6.0 %		—	—		87	4.1
Non-GAAP Income from Operations	\$	22	2.8 %	\$	207	27.7 %	\$	618	26.3 %	\$	583	27.7 %

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(4) 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP Diluted EPS	\$ (0.16)	\$ 0.55	\$ 2.04	\$ 1.56
Adjustments				
Stock-based compensation expense	\$ 0.25	\$ 0.22	\$ 0.69	\$ 0.76
Amortization of intangible assets	\$ 0.03	\$ 0.03	\$ 0.08	\$ 0.17
Acquisition-related costs ⁽²⁾	\$ 0.08	\$ —	\$ 0.08	\$ —
Gain on sale of nonfinancial assets ⁽³⁾	\$ —	\$ —	\$ —	\$ (0.05)
Severance and restructuring costs ⁽⁴⁾	\$ —	\$ 0.22	\$ —	\$ 0.44
Loss on investments ⁽⁵⁾	\$ —	\$ —	\$ 0.02	\$ 0.03
Income tax effect of adjustments	\$ (0.08)	\$ (0.11)	\$ (0.19)	\$ (0.31)
Non-GAAP Diluted EPS ⁽⁶⁾	\$ 0.12	\$ 0.91	\$ 2.69	\$ 2.60

(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 severance and restructuring costs related to the Company's 2024 corporate initiatives and the associated organizational redesign efforts.

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

(6) Reconciliation in slide 26.

Reconciliation of GAAP Weighted Average Diluted Shares Outstanding to Non-GAAP Weighted Average Diluted Shares Outstanding⁽¹⁾

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP Weighted-Average Diluted Shares Outstanding	192.0	197.1	196.9	196.7
Adjustments				
Common stock issuable under the company's equity plans ⁽¹⁾	0.8	—	—	—
Non-GAAP Weighted-Average Diluted Shares Outstanding	<u>192.8</u>	<u>197.1</u>	<u>196.9</u>	<u>196.7</u>

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