Delivering the next stage of BioMarin's growth

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Jefferies Healthcare Conference
November 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, Non-GAAP Operating Margin, operating cash flow and revenue compound annual growth rate (CAGR) for, in certain instances, 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 Metabolic Conditions portfolio, including PALYNZIQ, GALAFOLD and POMBILITI + OPFOLDA, and VOXZOGO; the anticipated benefits of the acquisition of Amicus Therapeutics, Inc. (Amicus), including the expected amount and timing of cost synergies as well as expected revenue from GALAFOLD and POMBILITI + OPFOLDA; the expected impact of the acquisition of Alesta Therapeutics B.V. and the BMN 450 license agreement; the anticipated benefits of the global settlement with Ascendis Pharma A/S, including BioMarin's expected receipt of royalty payments on net sales of Yuviwel; 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, PDUFA target action dates and label expansions; the expected benefits and availability of BioMarin's commercial products and product candidates, including with respect to the potential new indication for VOXZOGO in hypochondroplasia; and potential growth opportunities and trends, including the assumptions and expectations regarding total addressable patient population (TAPP) and market size 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 November 2, 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, 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, stock-based compensation expense and, in certain periods, certain other specified items, as detailed below when applicable. The company also includes a Non-GAAP adjustment for the estimated tax impact of the reconciling items. Non-GAAP Operating Margin percentage is defined by the company as GAAP Income (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. Non-GAAP synergies is defined by the company as Amicus' legacy labor and external spend cost reductions, excluding the impact of stock-based compensation. 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 to this presentation and BioMarin's quarterly earnings releases for the reconciliations to the comparable information reported under U.S. GAAP.

BioMarin: Diversified, Global Rare Disease Leader Operating at Scale

Focused on delivering medicines for people living with genetically defined conditions

25+
years of rare disease innovation

9
commercial therapies: 6 first-in-disease

4
on-market products with potential to reach $1B+ in annual revenue

~80
countries in our commercial footprint

$3.8–3.9B
FY'26 revenue guidance; 20% Y/Y growth expected at the midpoint

4
clinical-stage pipeline assets with data expected in 2027+

WHAT SETS BIOMARIN APART

Financial profile enables innovation
Strategic pipeline expansion driven by strong growth of revenue, earnings per share (EPS) and cash flow

End-to-end global capabilities
Discovery through development, manufacturing, regulatory and commercialization

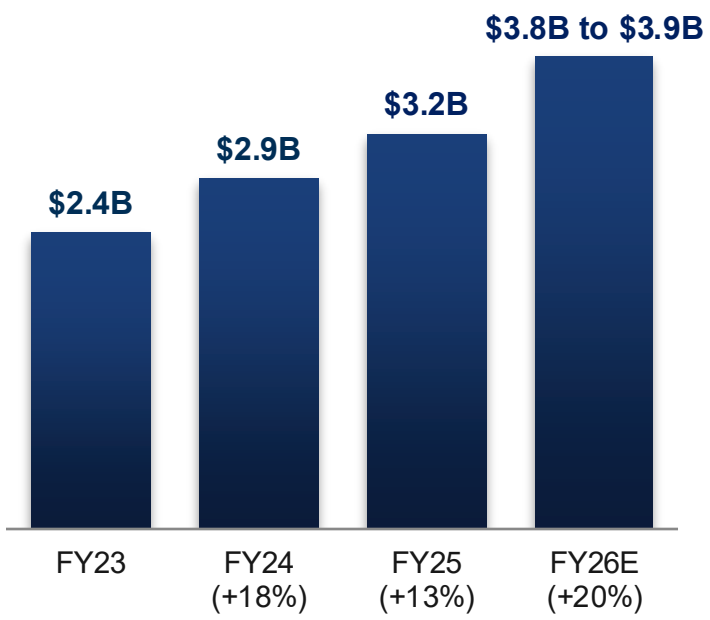
Finding, starting and keeping patients
Two decades of driving diagnosis and supporting patients worldwide

Three Years of Disciplined Execution Built a Profitable Growth Engine

Growing revenue, earnings per share (EPS), and cash flow enable strategic expansion

ANNUAL REVENUE

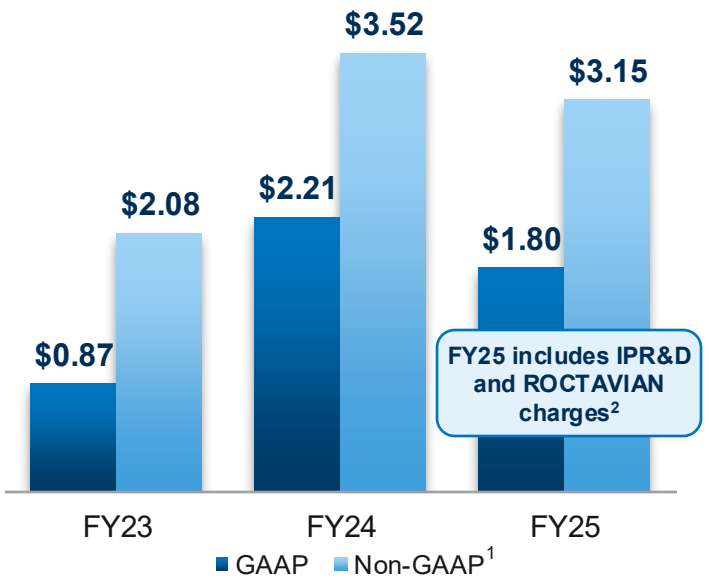
~60% expected growth
FY23 to FY26E guidance midpoint



Revenue ($B)

DILUTED EPS

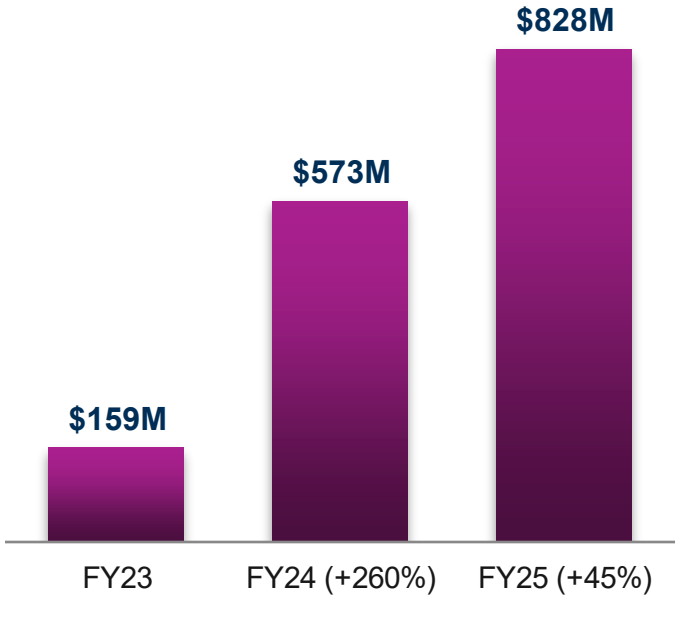
GAAP: > 100%
Non-GAAP[1]: > 50%
FY23 to FY25 increase



Diluted EPS ($)

OPERATING CASH FLOW

> 5x increase
FY23 to FY25



Operating Cash Flow ($M)

FY23 to FY25 reported total revenues; percentages show Y/Y change. [1]Refer to slide 2 for more detail on Non-GAAP financial measures. [2]2025 GAAP and Non-GAAP Diluted EPS include acquired in-process research & development charges related to the acquisition of Inozyme and charges related to the company's strategic decision to voluntarily withdraw ROCTAVIAN from the market.

Accelerating Growth with GALAFOLD and POMBILITI + OPFOLDA

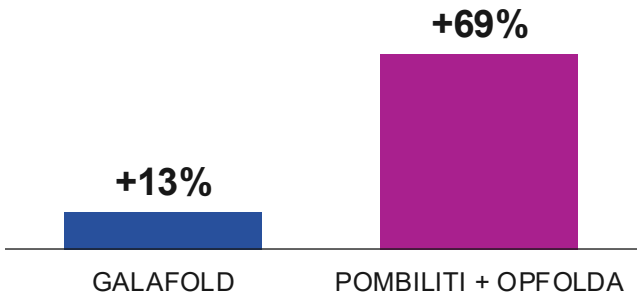
Amicus Acquisition Expected to be Accretive in First 9 Months Post-close (April 2026)

1 Strong Q3'26 Performance

$201M

combined GALAFOLD and POMBILITI + OPFOLDA revenue

Q3'26 patients treated, Y/Y growth



2 Cost Synergies[1,2] Expected to Drive Profitability

~$220M[2]

Non-GAAP cost synergies expected to be fully realized in 2028

- Cost synergies realization tracking ahead of expectations in 2026
- Substantial Non-GAAP Diluted EPS[1] accretion expected beginning in 2027
- Significant increase in operating cash flow anticipated

3 Peak Revenue Potential > $1B Each

$1.4B
GALAFOLD
by mid-2030s
(~10% CAGR '27-'32)

$1.2B
POMBILITI + OPFOLDA
by mid-to-late-2030s
(≥ 20% CAGR '27-'32)

GALAFOLD and POMBILITI + OPFOLDA Combined Non-GAAP Operating Margin[1]

> 60%

by 2030

All forward-looking figures are company estimates based on current portfolio. All figures represent additive contribution from Amicus acquisition. [1]Refer to slide 2 for more detail on Non-GAAP financial measures.
[2]Represents approximately $280 million of cost reductions on a GAAP basis, and approximately $220 million of cost reductions on a Non-GAAP basis, expected to be fully realized in 2028.

Pipeline Expansion Opens Major Market Opportunities

Four clinical programs with potentially differentiated profiles in large rare disease markets

LATE-STAGE | PHASE 3

BMN 820

FSGS[1]

~30K

Addressable U.S. patients[2]; BioMarin holds exclusive U.S. rights to program

PROFILE

- Potential to treat broad FSGS population, regardless of nephrotic syndrome status
- Phase 3 fully enrolled; proteinuria endpoint agreed with FDA

Potential first-in-class oral CCR2 inhibitor

Phase 3 data expected 2028

CLINICAL | PHASE 2/3

BMN 333

Achondroplasia (ACH)

~24K

Global total addressable patient population[2]

PROFILE

- Phase 1: free CNP exposure at highest dose >13-fold that of another long-acting CNP agent
- Phase 2/3 designed to show superiority vs. VOXZOGO

Potential next-generation long-acting CNP
Best-in-disease potential

Phase 2 data expected 2027

CLINICAL | PHASE 1/2a

BMN 440

Hypophosphatasia (HPP)

~35K

Global total addressable patient population[2]

PROFILE

- Potential first oral therapy; ENPP1 inhibitor acting upstream of PPi
- Systemic small molecule; preclinical data suggest reach beyond bone, such as muscle

First-in-disease potential in adult-onset HPP (U.S. and Europe)

Phase 1/2a data expected 2027

CLINICAL | PHASE 1/2

BMN 450

Fabry disease

~18K

~18K diagnosed worldwide; ~50% to 65% not amenable to GALAFOLD[2]

PROFILE

- Long-acting subcutaneous ERT
- Potential to treat all ERT-eligible Fabry patients
- Differentiated preclinical efficacy in renal, vascular and nerve tissue

Potential first subcutaneous ERT

Phase 1/2 study ongoing

[1]FSGS = Focal segmental glomerulosclerosis. [2]Company estimates.

Catalyst-rich 2027 Sets Up Next Phase of Growth

Expected data readouts and potential regulatory approvals

SKELETAL CONDITIONS			
BMN 440 (ALE1)	Phase 1/2a data for hypophosphatasia (HPP)	**DATA READOUT**	**2027**
BMN 333	Phase 2 dose-response data for achondroplasia	**DATA READOUT**	**2027**
VOXZOGO	Potential full approval for achondroplasia (PDUFA Feb. 28)	**REGULATORY**	**2027**
VOXZOGO	Potential U.S. and EU approval for hypochondroplasia	**REGULATORY**	**2027**
VOXZOGO	Phase 2 data for idiopathic short stature	**DATA READOUT**	**2027**

METABOLIC CONDITIONS & OTHER RARE DISEASES			
BMN 351	Phase 1/2 data for Duchenne muscular dystrophy	**DATA READOUT**	**2026**
POMBILITI + OPFOLDA	Potential adolescent approval for LOPD (PDUFA Jan. 10)	**REGULATORY**	**2027**
BMN 450 (HM15421/GC1134A)	Phase 1/2 study ongoing for Fabry disease	**PROGRAM ONGOING**	**2027**
BMN 430 (for ReNU syndrome)	Lead candidate selection underway	**PROGRAM ONGOING**	**2027**
BMN 820 (DMX-200)	Phase 3 data for FSGS	**DATA READOUT**	**2028**

Includes both ongoing and anticipated R&D highlights (non-exhaustive). PDUFA = FDA PDUFA target action date. LOPD = Late-onset Pompe disease.

Q&A

Thank you

Purpose-Driven. Patient-Focused. Future-Ready.

Appendix

Commercial Update Slides from BioMarin's Third Quarter 2026 Financial Results Presentation

As Presented on November 2, 2026

Strong GALAFOLD and POMBILITI + OPFOLDA Patient Growth in Q3'26

Galafold® (migalastat)

- **+13%** Y/Y patient growth
- **$152M** Q3 revenue
- **Expanded diagnosis and treatment**, with deeper penetration across established and newer markets
- **In international markets,** increased KOL engagement and family cascade testing contributed to patient growth

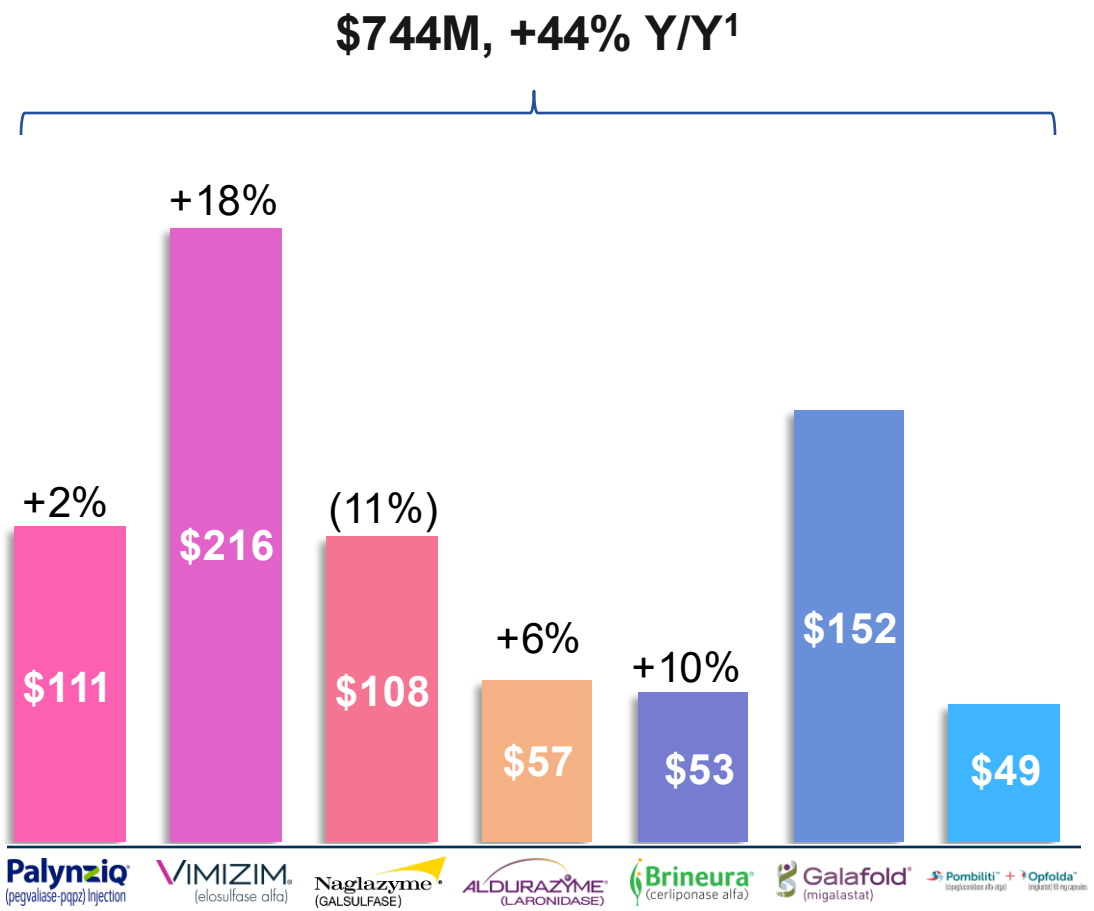
Pombiliti™ (cipaglucosidase alfa-atga) + Opfolda™ (miglustat) 65 mg capsules

- **+69%** Y/Y patient growth
- **$49M** Q3 revenue
- **Patient switching supported by a differentiated clinical profile** and growing clinical and real-world evidence, including five-year PROPEL data
- **Underlying growth complemented** by new patient starts due to supply constraints for other Pompe therapies

Metabolic Conditions: Diversified Portfolio Drove Record Results

Metabolic Conditions Q3'26 Revenue

In millions, except percentages



Q3'26 Dynamics

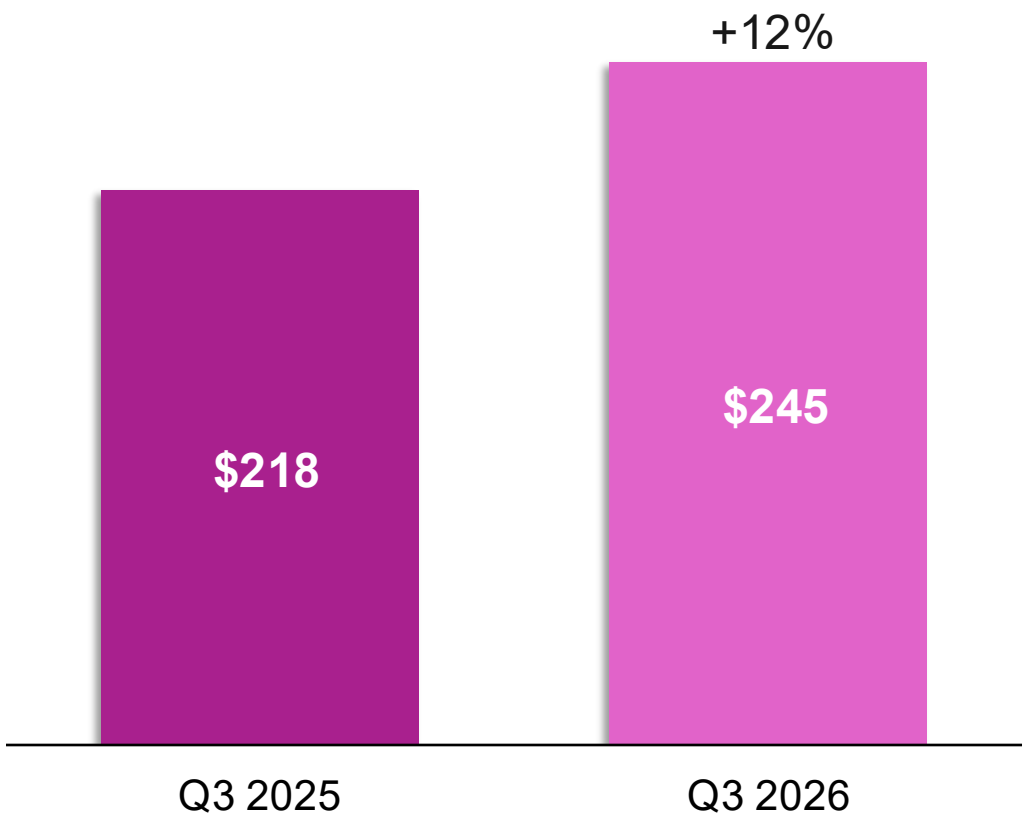
- **+44%** Y/Y revenue growth, driven largely by the first full quarter of GALAFOLD and POMBILITI + OPFOLDA
- Patients on therapy increased across all BioMarin-marketed Metabolic Conditions products, Y/Y
- The number of patients on PALYNZIQ increased 9% Y/Y, led by adolescent launch in U.S.
- VIMIZIM revenue increased 18% and NAGLAZYME decreased (11%) Y/Y, reflecting international order timing

[1]Year-over-year revenue comparisons are not available for GALAFOLD and POMBILITI + OPFOLDA because the prior-year periods occurred before BioMarin's acquisition of Amicus Therapeutics

Skeletal Conditions: Global Growth and Continued Leadership

VOXZOGO® Revenue

In millions, except percentages



Q3'26 Dynamics

- +12% Y/Y revenue growth; ~17% increase in the number of children treated globally Y/Y
- > 75% of revenue generated outside of the U.S.
- Large international order previously expected in Q3 now anticipated in Q4
- Children < 2 y/o represented majority of U.S. patient starts

HCH Launch Preparations

- ~14,000 total addressable patient population (TAPP)
- Focused on sharing strong evidence, increasing diagnosis and patient identification, and laying a foundation for rapid access
- HCH diagnosis to benefit from recent international consensus recommendations published in August 2026 in Nature Reviews Endocrinology[1]

Percentages reflect year-over-year change; [1]Dauber, A., Cheung, M.S., Hoover-Fong, J. et al. Diagnosis of hypochondroplasia: international Delphi consensus recommendations. Nat Rev Endocrinol (2026). https://doi.org/10.1038/s41574-026-01296-z

Appendix

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

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

(In U.S. dollars)

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

(1) Certain amounts may not sum or recalculate due to rounding.
(2) Represents a payment triggered by a third party's attainment of a regulatory approval milestone related to previously sold intangible assets.
(3) These amounts represent severance and restructuring costs related to the company's 2024 portfolio strategy review and the associated organizational redesign efforts announced in the second and third quarters of 2024. These amounts also include impairments of certain right-of-use and fixed assets.
(4) Represents the write-off of capitalized tooling and fixed assets in SG&A associated with the company's decision to cease development of the first generation VOXZOGO pen device in the fourth quarter of 2023.
(5) The current period represents a downward adjustment to non-marketable equity securities recorded in Other income (expense), net in the first quarter of 2024. The prior year represents the impairment losses on an investment in non-marketable equity securities and a convertible note recorded in Other expense, net in the first and fourth quarter of 2023, respectively.

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

(In U.S. dollars)

	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	$ 0.46	$ 0.92	$ 3.15	$ 3.52

(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.