44th Annual
J.P. Morgan Healthcare Conference

BIOMARIN®

Alexander Hardy
Chief Executive Officer

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BioMarin Pharmaceutical Inc.

January 12, 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 or the company), including, without limitation, statements about: preliminary estimated unaudited financial information for the year ended December 31, 2025, including total revenues and revenues from sales of VOXZOGO, as well as the expected asset write-down related to ROCTAVIAN and the related impact to BioMarin's non-GAAP financial results; expectations related to the consummation of BioMarin's proposed acquisition of Amicus Therapeutics and the anticipated benefits of such proposed acquisition, including strategic fit, acceleration of growth and diversification of revenue, and expectations of substantial accretion beginning in 2027; BioMarin's future financial performance, including the expectations of Total Revenues, revenue growth rates, and Non-GAAP Diluted EPS for, in certain instances, the full-year 2026 and future periods, and the underlying drivers of those results, such as the revenue opportunity represented by treatments for Skeletal Conditions, namely VOXZOGO and BMN 333, the expected demand and continued growth of BioMarin's Enzyme Therapies portfolio, including PALYNZIQ and the potential addition of adolescent age label expansion, as well as the planned expansion of and revenue opportunity represented by Galafold and the planned expansion, accelerated global launch and revenue opportunity of Pombiliti and Opfolda; 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, approvals, and label expansions; BioMarin's plans for pipeline expansion through external innovation; plans regarding ROCTAVIAN; plans to expand VOXZOGO for achondroplasia, as well as plans to add a second indication for hypochondroplasia in 2027 following the expected release of pivotal data with VOXZOGO in hypochondroplasia in the first half of 2026; expectations for BMN 333's efficacy compared to VOXZOGO's and ability to set a new standard of treatment for achondroplasia; 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, without limitations, risks relating to the completion of BioMarin's normal quarterly and annual accounting and financial statement closing procedures for the quarter and the year ended December 31, 2025; the timing of orders for commercial products; BioMarin's ability to meet product demand; risks and uncertainties with respect to the consummation of the proposed acquisition of Amicus Therapeutics and BioMarin's ability to obtain financing in the anticipated timeframe, if at all, including whether Amicus Therapeutics' stockholders will approve the acquisition, the possibility of competing offers, the possibility that various closing conditions for the transaction may not be satisfied or waived; the effects of the proposed acquisition on BioMarin's stock price and/or operating results, BioMarin's ability to realize the anticipated benefits of the proposed acquisition, including risks relating to integration, BioMarin's evaluation of the potential impact of the transaction on its financial results and financial guidance; the effects of the transaction on relationships with key third parties, including employees, customers, suppliers, other business partners or governmental entities, risks that the proposed transaction diverts management's attention from ongoing business operations, among others; and those factors detailed in 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.

Unaudited, Preliminary Financial Results

The financial information provided for the year ended December 31, 2025 is unaudited, preliminary, and subject to BioMarin's normal quarterly and annual accounting and financial statement closing procedures. Such information does not present all information necessary for an understanding of BioMarin's results of operations for the fiscal year ended December 31, 2025, and should not be viewed as a substitute for full financial statements prepared in accordance with U.S. generally accepted accounting principles. There can be no assurance that actual results will not differ from the preliminary estimates in this presentation. BioMarin expects to report its results for the fourth quarter and full year 2025 in February 2026.

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. For the quarter and year ended December 31, 2025, these other items are expected to include approximately $120 to $125 million of the asset write-down related to ROCTAVIAN. 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 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 has not provided a reconciliation of non-GAAP EPS to the most directly comparable GAAP reported financial measure because the company has not yet completed its normal quarterly and annual accounting and financial statement closing procedures and is unable to predict with reasonable certainty certain GAAP items, such as 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 our GAAP reported results for the period. As such, any reconciliations provided would imply a degree of precision that could be confusing or misleading to investors.

BioMarin's Unique Capabilities & Profile Unlock Long-term Value

Pioneering Innovation in Rare Disease

10

Marketed products[1]

6

Indications with **first-approved** treatment

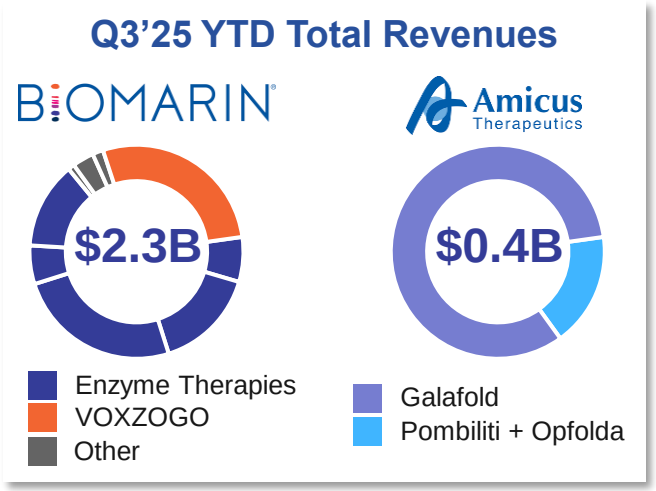
A leader in **genetically-defined conditions**

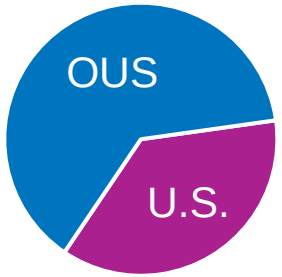
Expertise **finding** and **treating** patients with rare diseases

Industry Leading End-to-end Capabilities

- 80-country presence
- Strong global regulatory approval track record
- Drug discovery through patient treatment
- In-house manufacturing

Operating Scale Drives Strong Financial Outlook





Diversified revenue base

BioMarin Q3'25 YTD net product revenue[2]

[1]Pending 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; [2]Product revenue by geography excludes ALDURAZYME (marketed by Sanofi)

OUR PURPOSE

Be the biotech leader that translates the promise of genetic discovery into medicines that make a profound impact on the life of each patient.




ALDURAZYME® (LARONIDASE)

Brineura® (cerliponase alfa)

KUVAN® (sapropterin dihydrochloride) Tablets or Powder for Oral Solution

Naglazyme® (GALSULFASE)

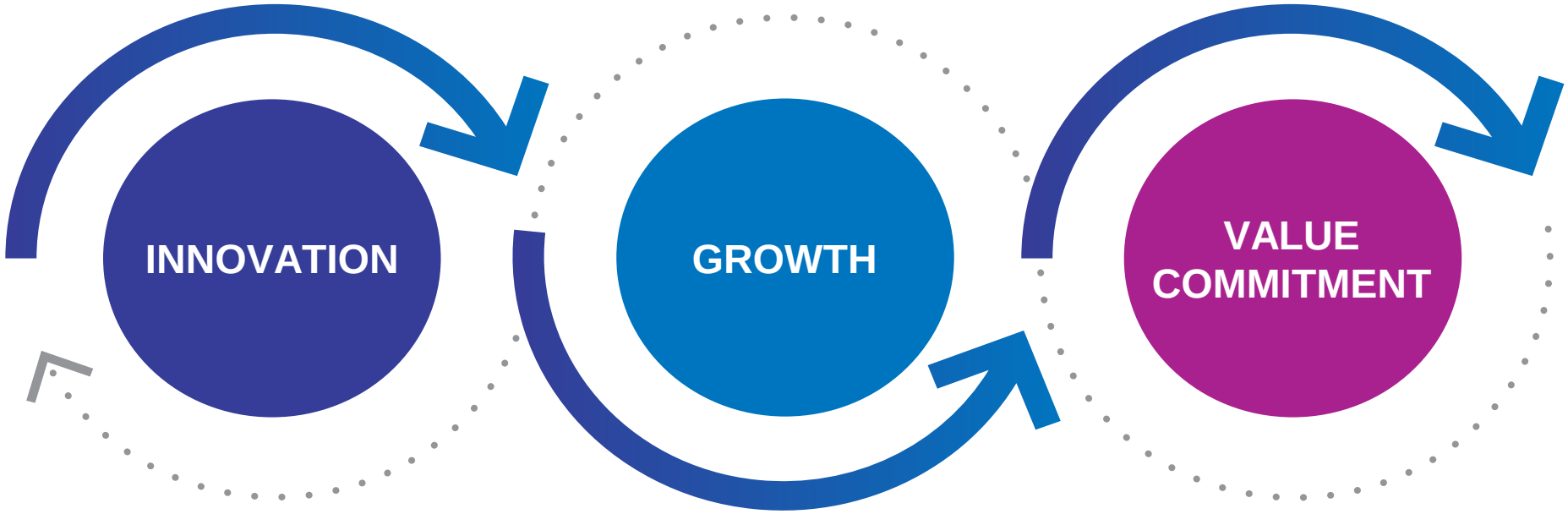
Palynziq® (pegvaliase-pqpz) Injection

ROCTAVIAN® (va.octocogene roxaparvovec-rvox)

VIMIZIM® (elosulfase alfa)

VOXZOGO® (vosoritide) for injection

Delivering on our Strategic Priorities over the Last 24 Months



- Executing on full lifecycle innovation opportunities
- Accelerated key assets (including BMN 333)
- Focused on mid- to long-term growth through internal & external innovation

- Achieved ~15% total revenue CAGR FY'23 – FY'25[1]
- Sustained high-single-digit growth of Enzyme Therapies
- VOXZOGO in achondroplasia now in 55 countries; expecting to add 2nd indication (hypochondroplasia) in 2027[2]

- Two M&A transactions in 2025[3]
- Increased profitability by > 3x compared to topline growth in FY'24, Y/Y
- Generated $728M operating cash flow Q3'25 YTD
- Targeting sustained double-digit long-term revenue CAGR

[1]CAGR calculated using unaudited preliminary revenue estimate of ~$3.2B for FY'25; [2]Pivotal data with VOXZOGO in hypochondroplasia 1H'26; Phase 2 proof-of-concept established; [3]Pending closure of BioMarin's acquisition of Amicus Therapeutics, expected to close in Q2'26, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions

2026 Priorities

Drive Accelerating Revenue

- VOXZOGO for achondroplasia & hypochondroplasia
- Enzyme Therapies and PALYNZIQ (12 to 17 y/o)
- Amicus Integration

Deliver a Catalyst-rich Year

- **2** Phase 3 data readouts; **1** filing for Full Approval
- **2** label expansions expected
- **2** data readouts to support program advancement

Strengthen the Pipeline

- Execution & advancement of existing assets
- Pipeline expansion from external innovation

December 19, 2025: BioMarin Announces Pending Acquisition of Amicus Therapeutics for $4.8 Billion, Expanding Leadership in Rare Diseases[1]

Exceptional Strategic Fit

- Opens access to BioMarin's 80-country footprint
  - Leveraging BioMarin's experienced field force
  - Accessing new countries
- Attractive R&D capabilities as well as opportunity for first-in-disease therapy with DMX-200
- Benefits from BioMarin's established manufacturing infrastructure

Accelerates & Diversifies Revenue

- Adds two high-growth products with multiple global expansion opportunities
- Expected to increase BioMarin's long-term revenue CAGR through 2030 and beyond
- Combined Galafold® and Pombiliti® + Opfolda® revenues totaled $599 million over the past four quarters ended Q3'25

Substantially Accretive Beginning in 2027

- Leveraging BioMarin's scale to deliver rapid accretion to Non-GAAP Diluted Earnings Per Share (EPS)[2]
- Drives significant cash flow to enable continuous reinvestment in innovation and growth
- Commitment to deleveraging, targeting gross leverage < 2.5x within two years after close

A Strategic Fit that Accelerates BioMarin's Growth Trajectory

[1]BioMarin's acquisition of Amicus is expected to close in Q2'26, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions;
[2]Refer to slide 3 for more detail on Non-GAAP financial measures

Plan to Expand Galafold Market with BioMarin Capabilities

Galafold® (migalastat) 123 mg capsule

The First and Only Oral Therapy Approved for Fabry Disease

Accelerate Penetration into Existing Markets

- Currently approved in > 40 countries, including Brazil and Turkey
- Established presence in large, underserved regions, Middle East and LatAm, to accelerate growth
- Direct BioMarin sales vs. distributors in majority of international markets

Increase Diagnosis and Treatment Rates

- Focus on diagnosis and treatment of Galafold-amenable patients (35-50% of diagnosed Fabry patients)
- 18,000 diagnosed worldwide; wide-ranging prevalence estimates
- Significant remaining unmet need; ~6k diagnosed untreated
- Leverage technology and family tree testing to increase diagnosis
- BioMarin's access pathways to accelerate treatment rates

Expand into BioMarin's Global Footprint

- Accelerate entry into new markets
- Global manufacturing capabilities and distribution channels to drive treatment rates in new countries
- Pricing and reimbursement expertise to facilitate access within BioMarin's 80 country footprint

Galafold represents a potential $1 billion peak revenue opportunity; FY'24 Galafold revenue $458M

Opportunity to Accelerate Global Launch of Pombiliti + Opfolda

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

The Only Two-component Therapy for Pompe Disease

Significant Expansion Opportunity Ahead

- Pombiliti & Opfolda early in global launch phase; approved in U.S. & EU ~2.5 years ago
- Reimbursed in 15 countries with major geographic expansion opportunity ahead
- New patient diagnosis rates projected to continue increasing
- Prescriber base increasing in breadth and depth

Strengthening Data Drives New Starts & Switches

- Growing body of real-world evidence supports switching from ERT-only therapies
- 50% of switch patients from ERT-only treatment had improved 6MWD or FVC[1]
- Switching increasing as ERT-experienced patients progress (after 2-3 years)
- Mechanistic differentiation and clinical data supports best in class profile

Exploring Indications to Address More Patients

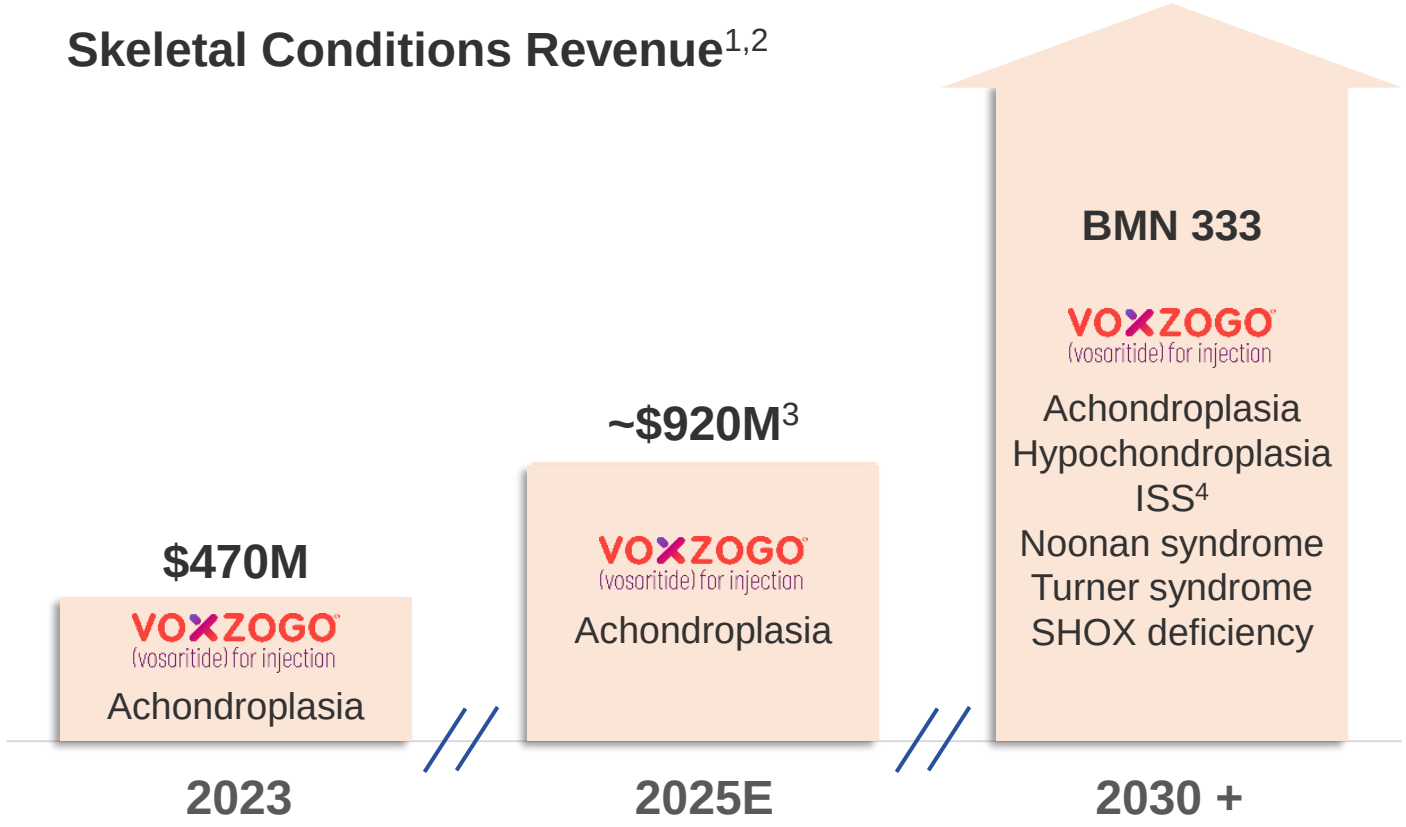
- Phase 3 study (ZIP) ongoing in children < 18 y/o with Late-onset Pompe Disease (LOPD)
  - Mid-2026: Potential approval for 12 to 17 y/o
  - Completing enrollment in < 12 y/o cohort
- Phase 3 study (ROSELLA) enrolling children < 18 y/o with Infantile-onset Pompe disease (IOPD)

Pombiliti + Opfolda represents a potential $1 billion peak revenue opportunity

[1]PROPEL MCID (Minimal clinically important distance) analysis; 6MWD: 6-minute walk distance; FVD: Forced vital capacity

2025 VOXZOGO Momentum Sets Stage for Skeletal Conditions Outlook

Skeletal Conditions Lifecycle Strategy to Drive Long-term Revenue Growth[1]



Building on VOXZOGO's Leadership in Achondroplasia

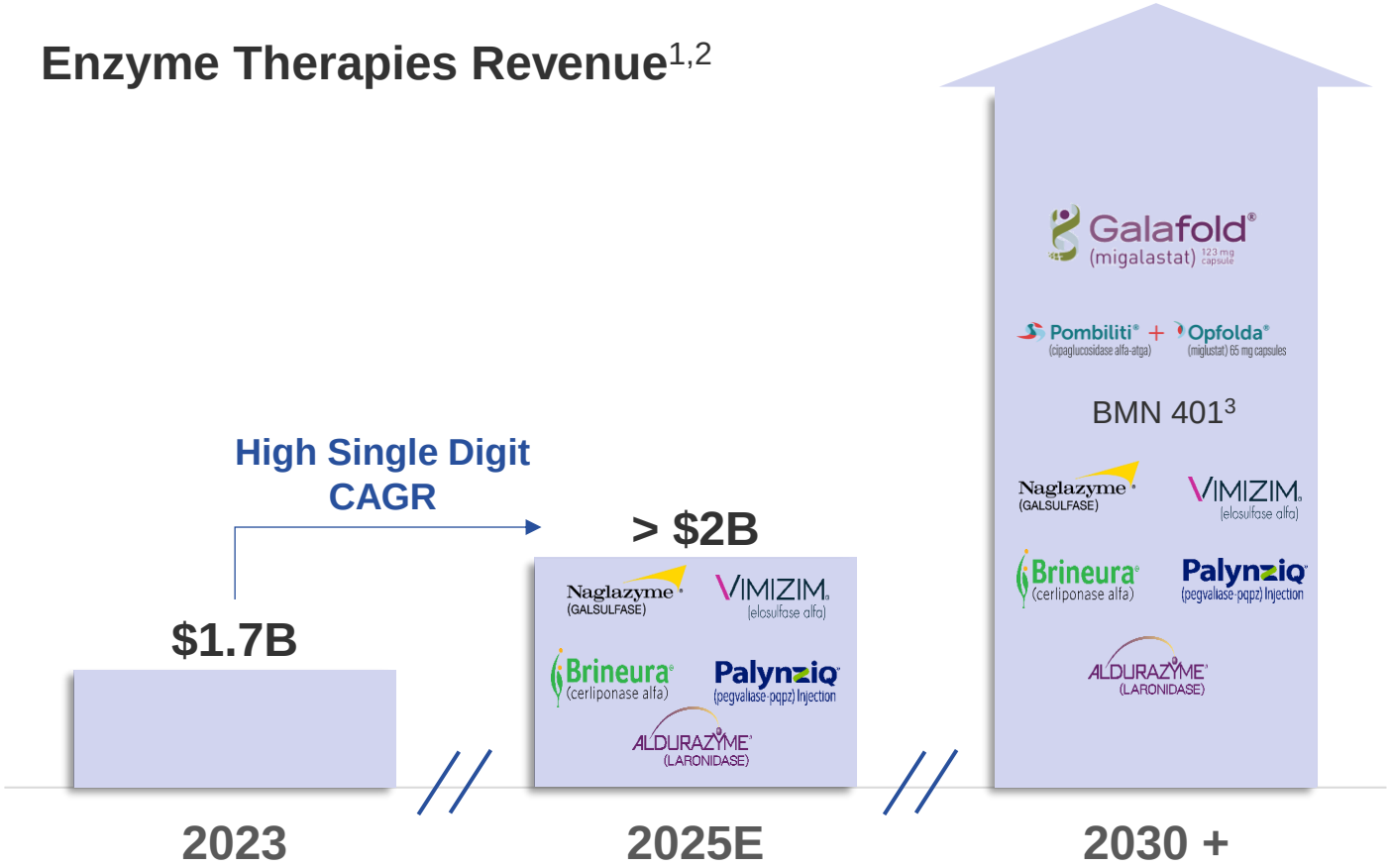
- 10,000 patient years of robust efficacy and safety data; significant evidence generated to support multiple benefits beyond height
- Only approved precision therapy for infants and children; available in 55+ countries
- Expected to be the only approved product for <2-year-olds through 2027
- Int'l treatment guidelines for VOXZOGO and growing body of clinical benefit
- Patent portfolio includes multiple variants of long-acting CNP across key geographies
- ITC case and ODE resolution expected in '26[5]

[1]Chart not drawn to scale; [2]BMN 333 and indications beyond achondroplasia pending supportive data from registrational studies and regulatory approval; [3]Unaudited preliminary 2025 revenue estimate; [4]ISS: Idiopathic short stature; [5]ITC: U.S. International Trade Commission; ODE: Orphan Drug Exclusivity

Accelerating Growth across Enzyme Therapies Business

Strong Enzyme Therapies Outlook

Enzyme Therapies Revenue[1,2]



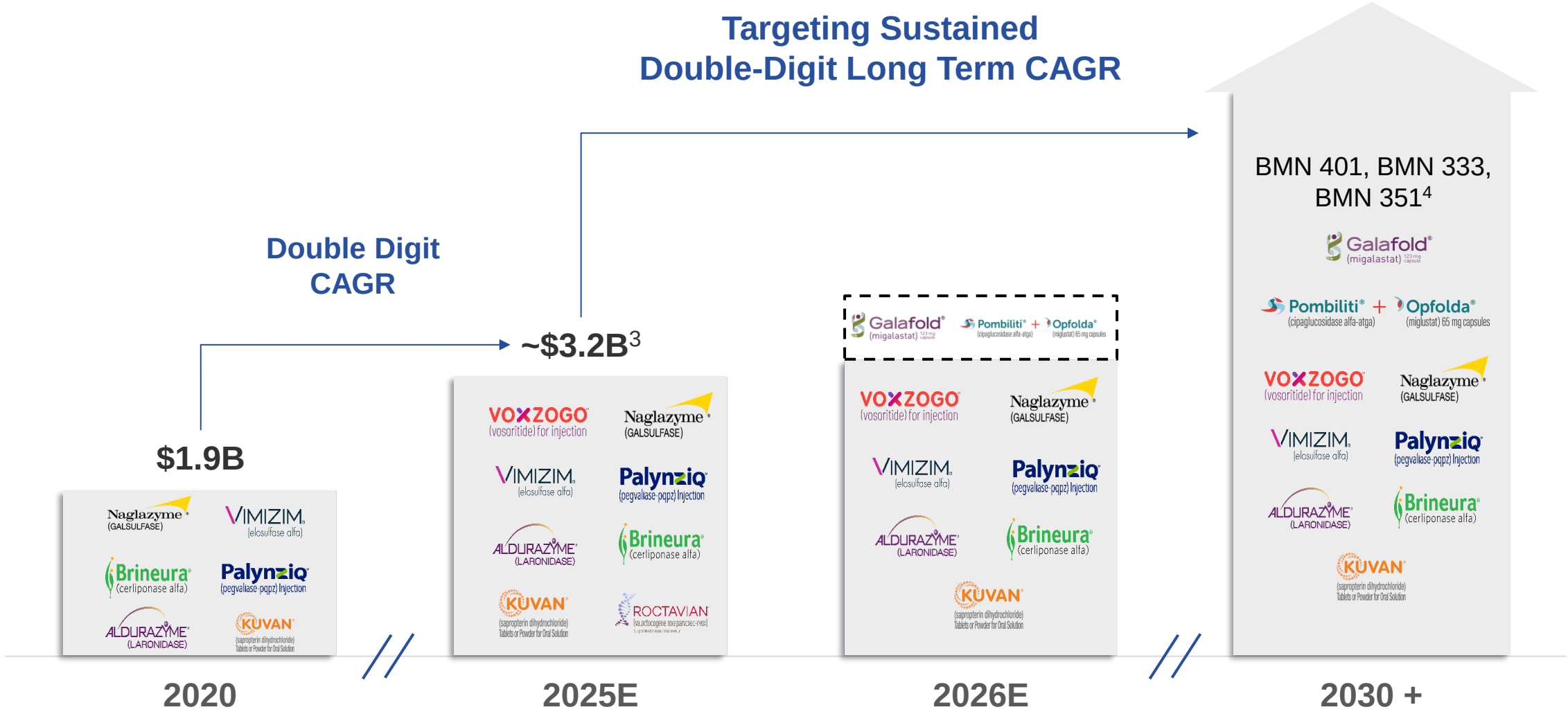
Key Strategies

- Continue to advance PALYNZIQ as a growth driver; potential to add adolescent age label expansion to increase eligible patients
- Leverage BioMarin's commercial and manufacturing capabilities to maximize value of Galafold and Pombiliti + Opfolda
- Unlock growth opportunities in emerging markets across the product portfolio
- Increase diagnosis rates to find more patients using broader testing methods, including family tree diagnostics
- Maintain high patient adherence rates across the Enzyme Therapies portfolio

[1]Chart not drawn to scale; [2]Contributions from Galafold & Pombiliti + Opfolda pending transaction closure with Amicus, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions; [3]Pending supportive data from registrational study and regulatory approval

Diverse and Growing Revenues Propel Strong Long-term Outlook

Total Revenues[1,2]



[1]Chart not drawn to scale; [2]Contributions from Galafold & Pombiliti + Opfolda pending transaction closure with Amicus, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions; [3]Unaudited preliminary 2025 revenue estimate; [4]Contributions from BMN 401, BMN 333, and BMN 351 subject to pending supportive data from registrational studies and regulatory approvals

BIOMARIN
2026

Deliver a Catalyst-rich Year

Research and Development Update

Greg Friberg
Chief R&D Officer

2026 Pipeline Highlights Across Therapeutic Areas

3 Major Data Readouts and Planned Submissions for Regulatory Approval

VOXZOGO (vosoritide) for injection
hypochondroplasia
(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: hypochondroplasia, idiopathic short stature, Noonan syndrome, Turner syndrome, SHOX deficiency

BMN 333
Long-acting CNP
New product for achondroplasia

2 Label Expansions Expected for Adolescents

Palynziq (pegvaliase-pqpz) Injection
PKU

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

2 Program Updates to Enable Registration-enabling Studies

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

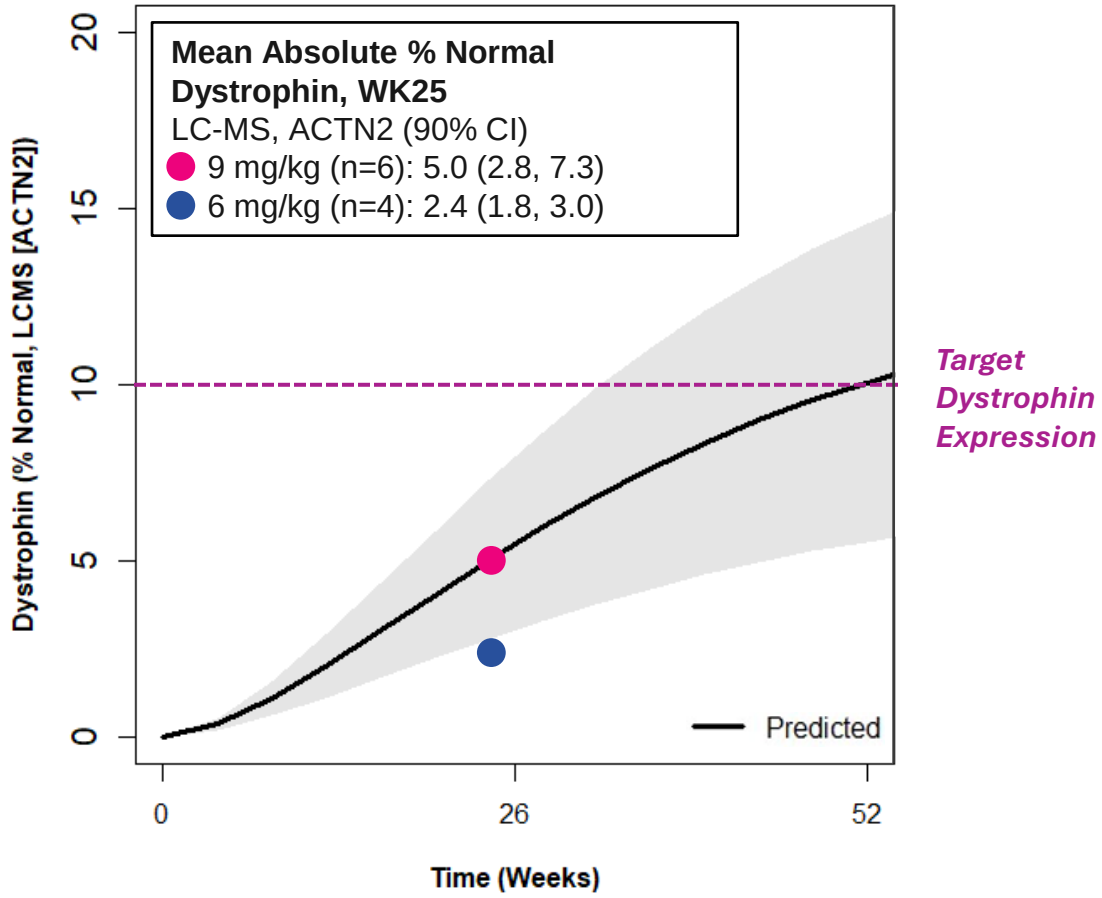
BMN 333
Long-acting CNP for achondroplasia
(Phase 1 data)

BMN 351: Encouraging Dystrophin Responses in First Two Cohorts

Study in Duchenne muscular dystrophy (DMD) continues as planned, with 12 mg/kg cohort biopsy data anticipated in 2H'26

Observed and Predicted Dystrophin Expression

LCMS Projections for Absolute Mean Dystrophin



Study 351-201: Key Highlights

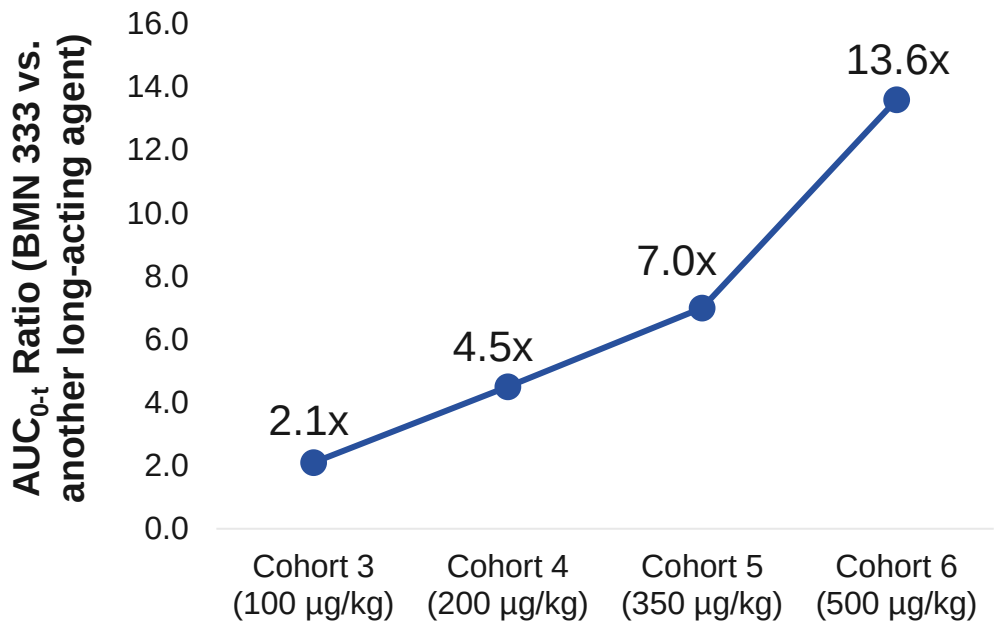
- **Clear dose-dependent dystrophin response** with encouraging levels measured at week 25 biopsy
- **Findings are consistent across multiple assays**
  - Liquid chromatography-mass spectrometry (LCMS), without histologic adjustment for muscle content (depicted on the left)
- **Differentiated phosphorothioate chemistry**
  - Long tissue half-life and continued dystrophin accumulation in muscle: ~2x @ WK52 vs. WK25
- **Study continues as designed to evaluate**
  - Dystrophin expression for 12 mg/kg cohort
  - Longer-term safety across all doses
  - Functional measures (including SV95C)
- Expect to present full results from both the 6 and 9 mg/kg cohorts at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference in March 2026

Modeled projections are for 9 mg/kg where gray area around the predicted value represents 90% confidence interval. Assumptions (e.g. drug, exon skip product, and dystrophin half-lives) are based on data from Del52/mdx mice, NHPs, and prior human experience in a phosphorothioate oligonucleotides study for DMD.

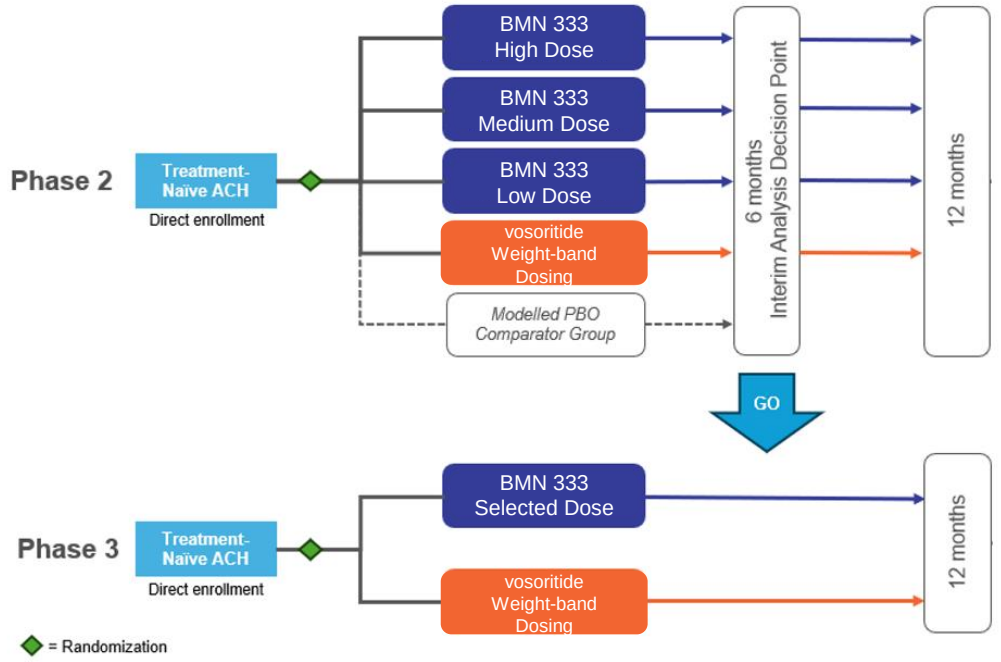
BMN 333: Early Results Support Initiation of Phase 2/3 Study in Children with Achondroplasia

*Exposure for free CNP (AUC) was **increased by over 13X** compared to another long-acting CNP agent[1]*

BMN 333 Surpassed 3x Free CNP Target in Phase 1 PK Study



BMN 333 Phase 2/3 Study Design[2] Enrollment to Begin 1H'26



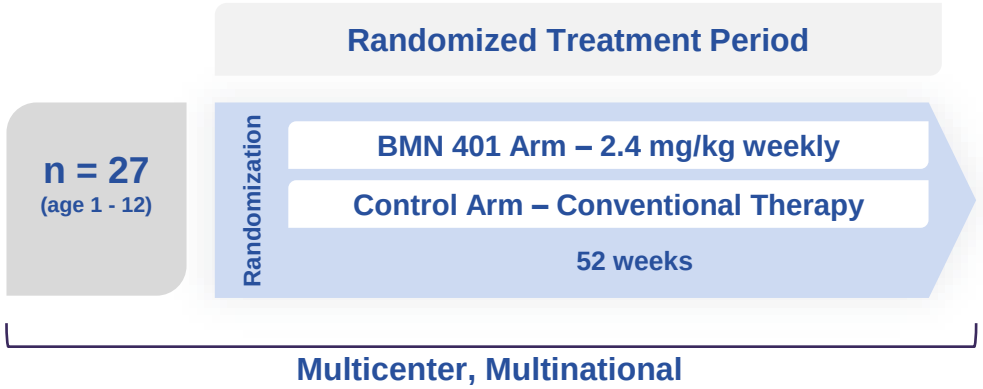
[1]Breinholt et al., Br J Clin Pharmacol. 2022;88(12):5203–5215. doi:10.1111/bcp.15369. (reference for geometric mean of $AUC_{0\text{-}t}$)
[2]Study design subject to change post-feedback from regulatory authorities

BMN 401: Potential 1st Disease Modifying Therapy for ENPP1 Deficiency

Pivotal Data Anticipated 1H'26

BMN 401 Phase 3 (ENERGY 3) Design

Design: Randomized (2:1), Open Label



Endpoints

- **Co-Primary**:
  - Change in plasma PPi from baseline over time
  - RGI-C score
- **Secondary**: RSS, Growth Z-score; PK

Key Highlights

- **Registration-enabling study with BMN 401 for ENPP1 deficiency** in children 1-12 y/o
- Skeletal improvements by RGI-C have an established track record in other genetic forms of Rickets
- Topline pivotal data expected **1H'26**
- Progressing plans to advance BMN 401 for ENPP1 deficiency across additional age groups
- Potential to address between **2-2.5k** people with ENPP1 deficiency in BioMarin's territories (includes all age groups)

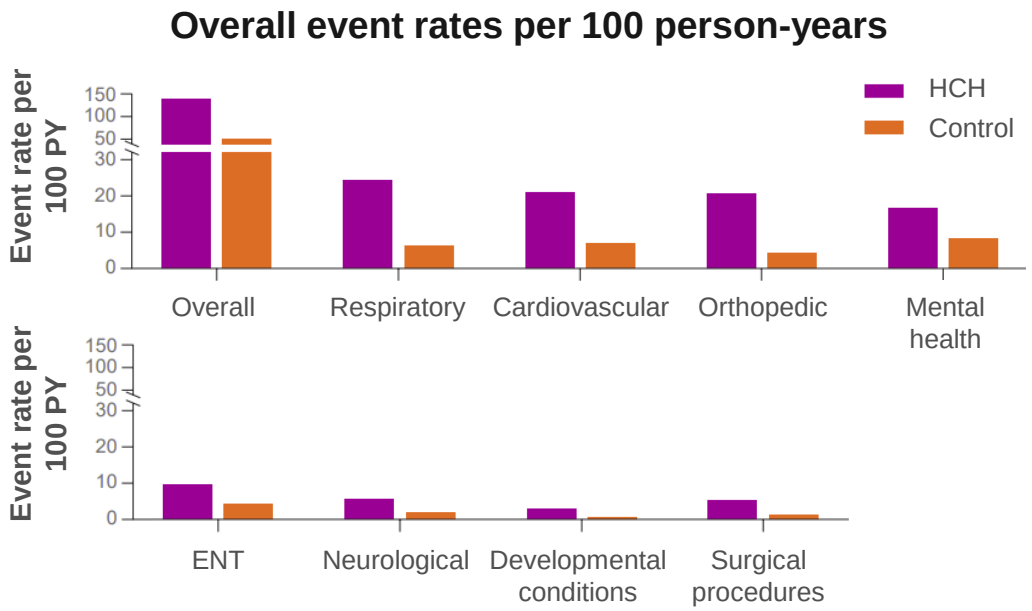
PPi: Plasma Inorganic Pyrophosphate; RGI-C: Radiographic Global Impression of Change; RSS: Rickets Severity Score; PK: Pharmacokinetic

Next Potential VOXZOGO Indication: Hypochondroplasia (HCH)

Pivotal Data Anticipated 1H'26

Significant Unmet Need in HCH

Comorbidity and surgical procedures in individuals with HCH vs controls



Key Highlights

- HCH prevalence: 4.2 / 100,000 individuals
- Total Addressable Patient Population: **14,000**
- Adults with HCH **showed higher mortality** compared with controls: (3.4 rate ratio), **similar to rates seen in achondroplasia**
- **Global initiatives to improve early HCH diagnosis**:
  - Clinician & community awareness
  - Targeted genetic reclassification
  - Diagnostic pathway optimization
- **VOXZOGO for HCH topline Phase 3 data expected 1H'26**
- Full data to be shared at a medical congress in **2H'26**

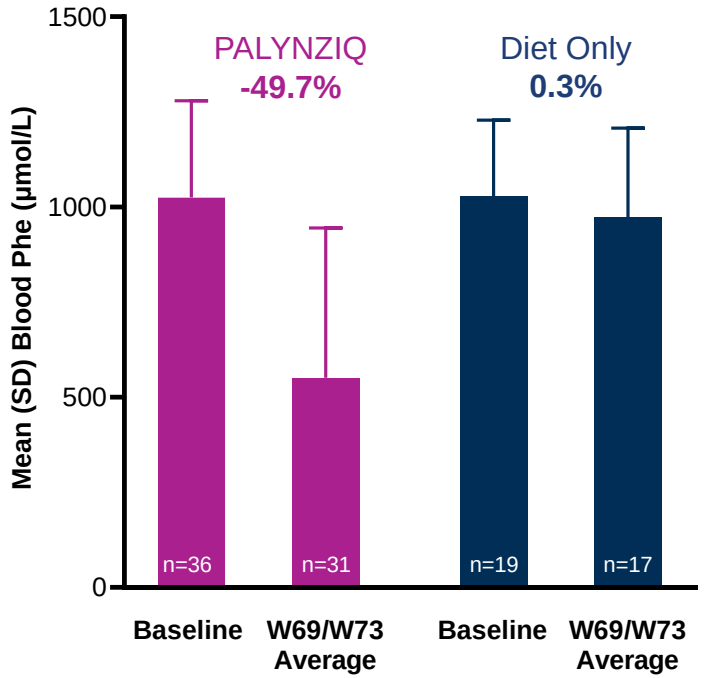
Mukherjee S et al. Medical Impact of HCH among children and adults in England: a 22-year retrospective study (poster)

The Unique Benefits of PALYNZIQ for Adolescents

FDA PDUFA Date of Feb. 28, 2026, for Potential Age Expansion (12-17 y/o)

PEGASUS Phase 3 Study of PALYNZIQ in Adolescents with PKU

Mean % Change from Baseline Following 72 Weeks on Study



Key Highlights

- **PEGASUS Results (week 72):**
  - **Statistically significant reduction in blood Phe** compared to diet-only (p<0.0001)
  - **~100%** ↑ in protein from intact food
  - Safety results consistent with known profile of PALYNZIQ
- Caregiver support during titration period with PALYNZIQ may drive adherence through adulthood
- Potential for adolescents treated with PALYNZIQ to increase share of “in-clinic” patients
- Anticipated U.S. and EU PALYNZIQ label expansion to adolescents in **2026**

Sacharow S, et al. Safety and efficacy of pegvaliase in adolescents with phenylketonuria: Primary results from PEGASUS, a phase 3 open-label randomized controlled study; PEGASUS study enrolled adolescents aged 12-17 years; Phe: phenylalanine; W: week

A Catalyst-rich Outlook for 2026

1H'26

Palynziq (pegvaliase-pqpz) Injection
PKU
Adolescents (12-17 y/o)
U.S. sBLA PDUFA

Palynziq (pegvaliase-pqpz) Injection
PKU
Adolescents (12-15 y/o[1])
EU Approval

BMN 401
ENPP1 deficiency
Phase 3
Topline Data

VOXZOGO (vosoritide) for injection
hypochondroplasia
Phase 3
Topline Data

BMN 333
achondroplasia
Phase 1 PK Data
(Medical Congress)

BMN 351
DMD
Phase 1/2
Full Results,
6 and 9 mg/kg
(MDA[2])

VOXZOGO (vosoritide) for injection
achondroplasia
sNDA Submission for
Full Approval

BMN 333
achondroplasia
Phase 2/3
Registration-enabling
Study Initiation

2H'26

VOXZOGO (vosoritide) for injection
hypochondroplasia
Phase 3 Full Data
(Medical Congress)

VOXZOGO (vosoritide) for injection
hypochondroplasia
Submissions for
Regulatory Approval

BMN 401
ENPP1 deficiency
Submissions for
Regulatory Approval

BMN 351
DMD
Phase 1/2
Topline Results,
12 mg/kg

Pombiliti® (cipaglucosidase alfa-atga) + **Opfolda®** (miglustat) 65 mg capsules
Pompe disease
Label Expansion
(12-17 y/o)[3]

- Skeletal Conditions
- Enzyme Therapies
- Pipeline

Illustrative view of anticipated pipeline events in 1H'26 and 2H'26; does not reflect specific dates; [1]PALYNZIQ is currently approved for patients aged 16 years and older in the EU; [2]MDA: Muscular Dystrophy Association; [3]Pending closure of BioMarin's acquisition of Amicus Therapeutics, expected to close in Q2'26, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions

Summary

Transformed BioMarin Ready for Next Phase of Growth

- ✓ Refined Revenue Excellence with Focused BU Operating Model
- ✓ Prioritized R&D Pipeline to Advance the Most High-impact Medicines
- ✓ Introduced $500M Cost Transformation

- ✓ Double Digit Revenue Growth
- ✓ Progressed Key Pipeline & Lifecycle Assets to Next Stage of Development
- ✓ Two Significant Public Company M&A Transactions[1]
- ✓ Consistent Expansion of Profitability and Cash Generation

- Targeting Sustained Double-Digit Revenue CAGR
- R&D Catalyst-rich Outlook Augmented by External Innovation
- Demonstrated Execution, Profitability Growth and Cash Management

[1]Pending closure of BioMarin's acquisition of Amicus Therapeutics, expected to close in Q2'26, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions

Thank you.

Q&A

Appendix

Preliminary Financial Update Today

Preliminary Financial Update Today

Item	Guidance from Q3'25 Earnings	Today's Update[1]
FY 2025 Revenue	$3,150M - $3,200M	~$3.2B
VOXZOGO Revenue	$900M - $935M	~$920M
Q4 ROCTAVIAN Asset Write Down (Non-GAAP Diluted EPS Impact)	Not Included	($0.60) – ($0.64)[2]

Notes

- Q4 Roctavian asset write-downs are estimated to be $230M – $260M and include inventory, facilities, intangible assets, and severance costs, of which approximately $120M – $125M, or ($0.60) – ($0.64) per share, related to inventory is expected to reduce Non-GAAP Diluted EPS.
- Non-GAAP Diluted EPS guidance, excluding ROCTAVIAN asset write-down impact, remains unchanged, as the expected revenue result is partly offset by incremental Q4 operating expense.
- ROCTAVIAN revenue to be excluded from 2026 guidance.

[1]Unaudited preliminary 2025 financial results; [2]Non-GAAP Diluted EPS assumes approximately 200 million Weighted-Average Diluted Shares Outstanding