43rd Annual
J.P. Morgan Healthcare Conference

BIOMARIN®

BioMarin Pharmaceutical Inc.

Alexander Hardy, President and Chief Executive Officer

January 13, 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, Operating Cash Flow and Revenue Compound Annual Growth Rate (CAGR) for future periods and the underlying assumptions; BioMarin's plans and expectations regarding innovation and growth, including plans regarding business development and clinical-stage external innovation, as well as the anticipated benefits of such plans; ability of BioMarin's approved products, including VOXZOGO and BioMarin's enzyme therapies, to drive long-term revenue growth; the clinical development and commercialization of BioMarin's product candidates and commercial products, including plans and expectations regarding (i) the ability to expand BioMarin's leadership in achondroplasia with VOXZOGO and leverage VOXZOGO in other skeletal conditions, including hypochondroplasia, idiopathic short stature, Noonan Syndrome, Turner Syndrome and SHOX deficiency; (ii) development of BMN 333 for the treatment of achondroplasia and hypochondroplasia, (iii) expansion of PALYNZIQ for the treatment of adolescents with phenylketonuria (PKU), (iv) development of BMN 390 for the treatment of PKU, (v) development of BMN 351 for the treatment of Duchenne Muscular Dystrophy, (vi) development of BMN 349 for the treatment of alpha-1 antitrypsin deficiency, and (vii) development of BMN 370 for the treatment of von Willebrand disease; the expected benefits and availability of BioMarin's product candidates and commercial products; the timing of BioMarin's clinical development and commercial prospects, including announcements of data from clinical studies and trials; potential growth opportunities and trends, including the assumptions and expectations regarding Total Addressable Patient Population with respect to the conditions targeted by BioMarin's product candidates and commercial products; and the legal action BioMarin initiated against Ascendis Pharma A/S at the Unified Patent Court for infringement of BioMarin's patent rights, including BioMarin's expectations regarding the timing and resolution of such legal action.

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 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, 2024 as such factors may be updated by any subsequent reports. You should carefully consider that information before you make an investment decision. You should not place undue reliance on forward-looking statements, which speak only as of the date hereof. These forward-looking statements are based on the beliefs and assumptions of the Company's management based on information currently available to management and should be considered in connection with any written or oral forward-looking statements that the Company may issue in the future as well as other cautionary statements the Company has made and may make. Except as required by law, BioMarin does not undertake 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 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 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. 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 Non-GAAP Income, Non-GAAP Operating Margin percentage, Non-GAAP Diluted EPS and Non-GAAP Weighted-Average Diluted Shares Outstanding are important internal measurements for BioMarin, the company believes that providing this information in conjunction with BioMarin's 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. BioMarin also uses Non-GAAP Income internally to understand, manage and evaluate its business and to make operating decisions, and compensation of executives is based in part on this measure.

Non-GAAP 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 22 for the reconciliations to the comparable information reported under U.S. GAAP.

BioMarin is the global leader in treating genetically defined conditions.

We pioneer new medicines through four connected strengths.

SCIENTIFIC EXCELLENCE

Uncovering and solving for the root cause of conditions

ACCELERATED INNOVATION

Rapidly transforming insights into category-defining medicines

IN-HOUSE EXPERTISE

Driving the development and manufacturing of complex medicines

GLOBAL DELIVERY

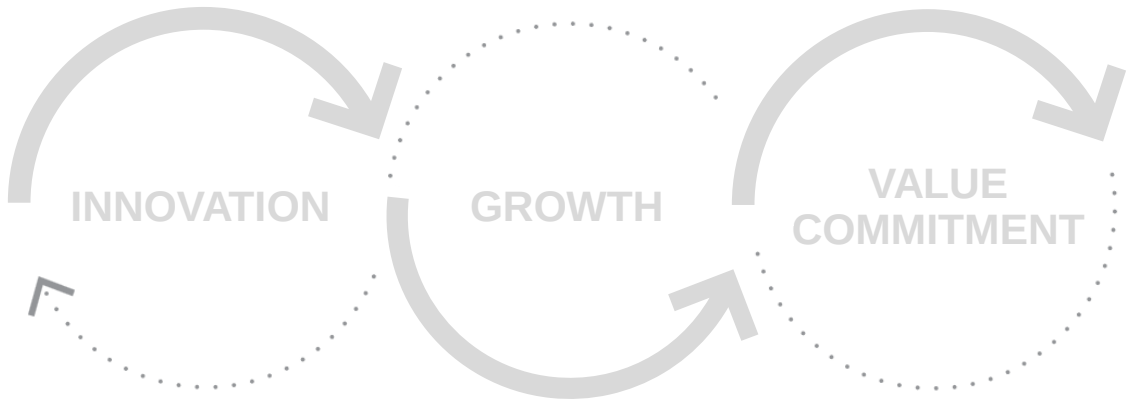
Ensuring life-changing products reach patients counting on us most

Building on Strong Execution in 2024

3Q'24 Year-to-date Financials

~$2.1B +19%	27.7% +7.6 ppts	$2.60 +63%
Total Revenues	Non-GAAP Operating Margin[1]	Non-GAAP Diluted Earnings Per Share[1]

New Strategy to Drive Value Creation



Milestones Anticipated over Next 18 Months

Skeletal Conditions
- 5 indications[2] advancing in VOXZOGO and BMN 333 PK data

Enzyme Therapies
- Palynziq (12-17 yrs) Phase 3 Data and U.S. & EU sBLA Filings
- BMN 390 (PKU with novel PEG) First-in-human study start

Pipeline
- BMN 351 (DMD[3]) Clinical POC Data; BMN 349 (A1AT[3]) Study Start

Prioritizing Business Development

Financial Outlook Provided through 2034

~$4B	Low-to-Mid 40%'s, starting with 40% in 2026	$1.25B+	Targeting Mid-teen CAGR
2027 Total Revenues	Non-GAAP Operating Margin[1]	2027 Operating Cash Flow	2023 – 2034 Revenue Growth[4]

[1]Refer to slide 3 for more detail on Non-GAAP financials; [2]Hypochondroplasia, Idiopathic Short Stature, Noonan Syndrome, Turner Syndrome, SHOX deficiency; [3]Duchenne Muscular Dystrophy, Alpha-1 Antitrypsin Deficiency; [4]Revenue growth shown on a non-risk-adjusted basis.

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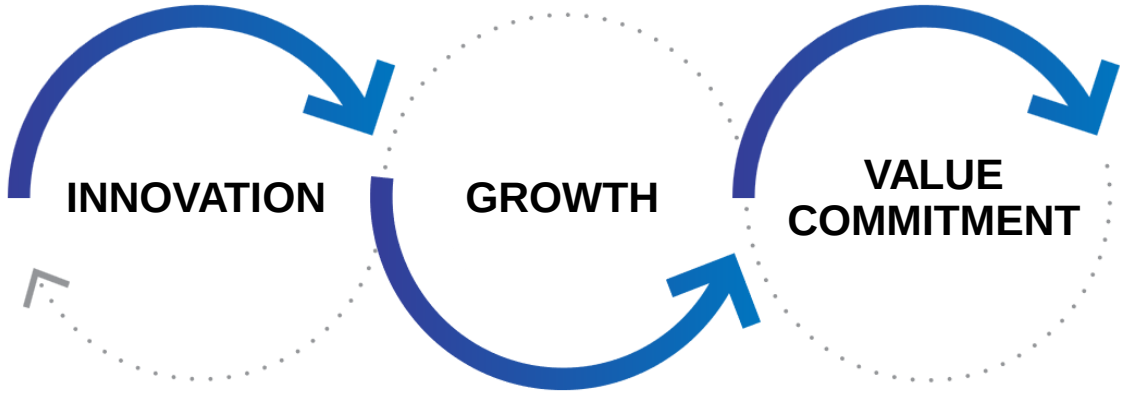

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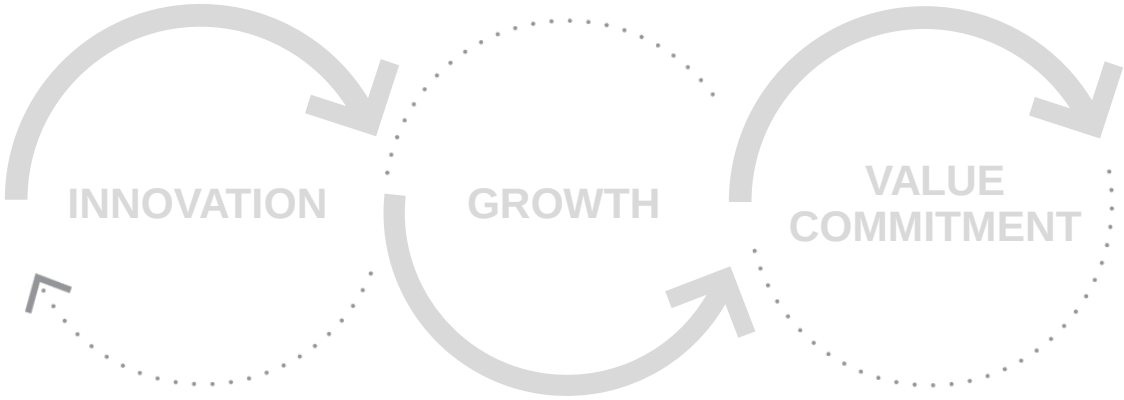

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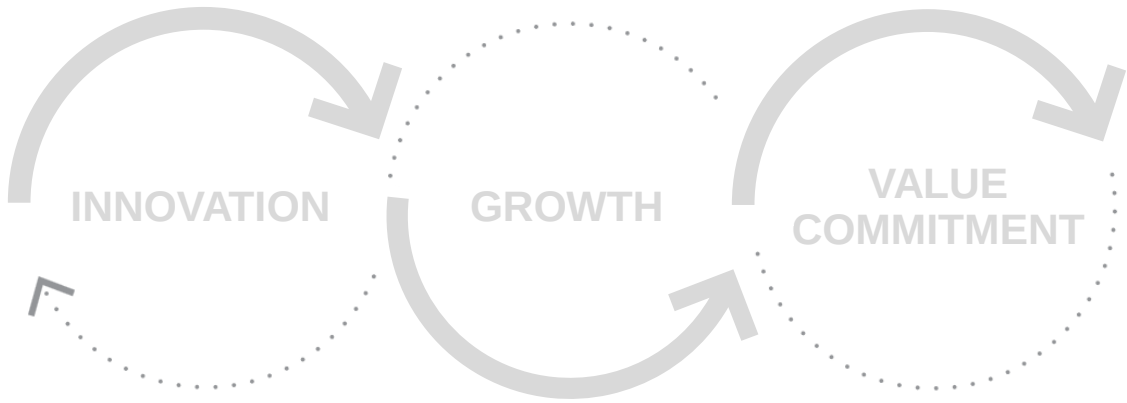

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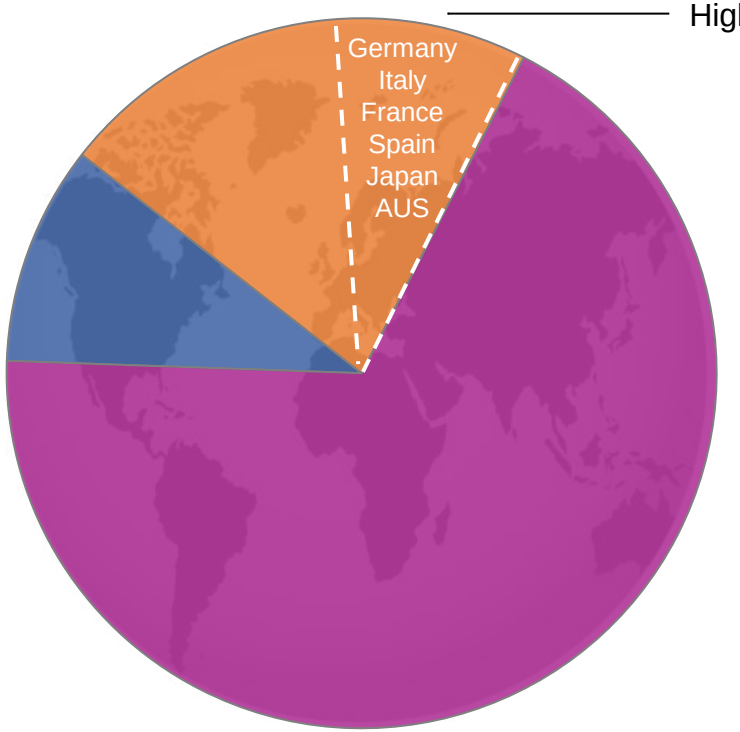
VOXZOGO Presents Attractive Global Expansion Opportunities with 90% of Total Addressable Patient Population (TAPP) Outside the U.S.

Percentage of Infants and Children with Achondroplasia Eligible for VOXZOGO Treatment; (24K Global TAPP)

Europe, Japan, Australia, Canada: ~22% of VOXZOGO TAPP

Highly Penetrated, Large Strategic Markets



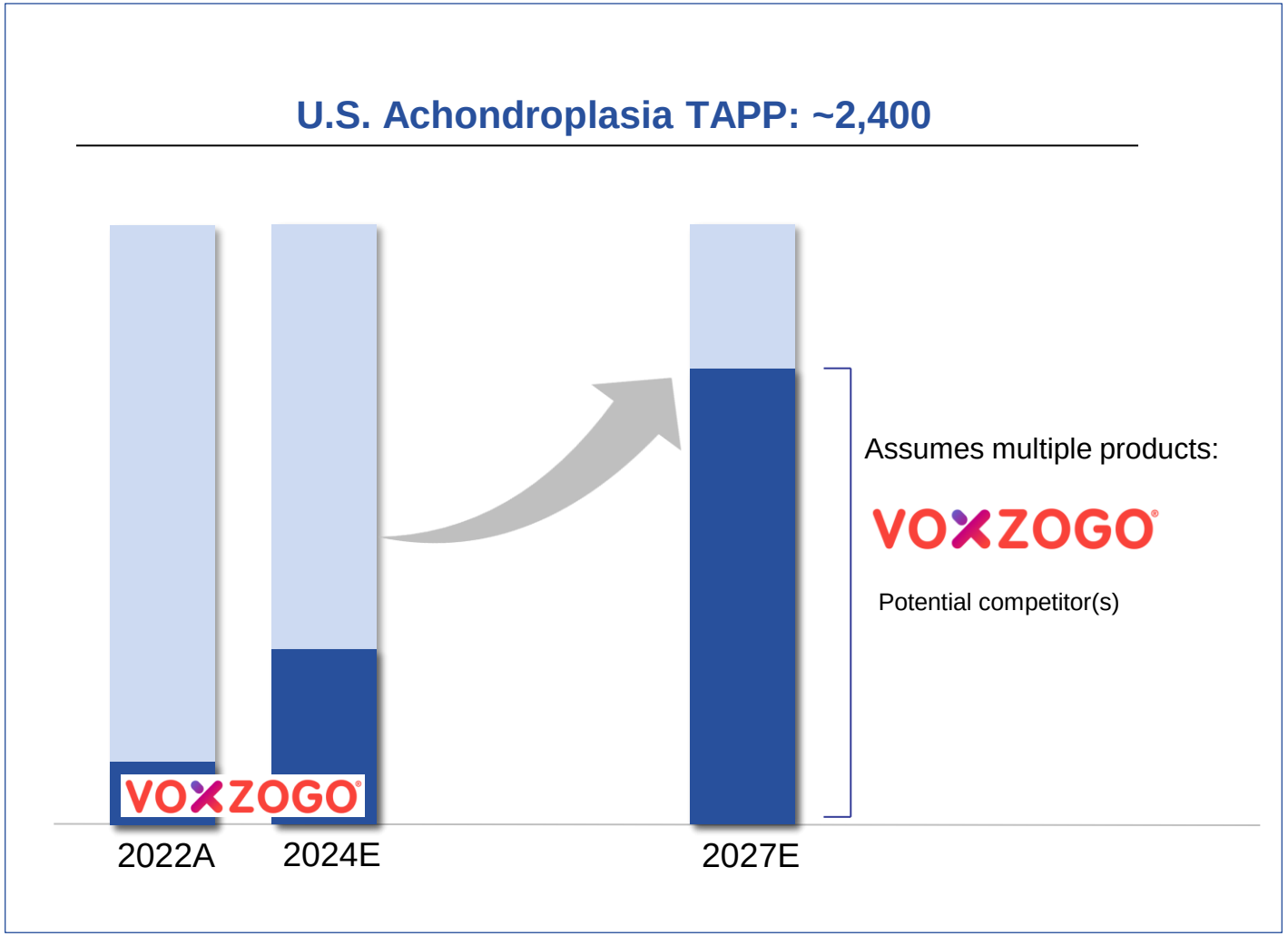
United States: ~10% of VOXZOGO TAPP

Rest of World: ~68% of VOXZOGO TAPP

- Leveraging 20-year global capabilities in patient finding, diagnosis, access, and support for first-in-therapy treatments
- VOXZOGO available in majority of ROW countries with plans to add remaining by 2027
- Applying learnings from successful ROW launches

TAPP defined as the diagnosed, clinically eligible patients for a given product or program in a defined footprint; footprint defined as all global markets included in BioMarin's internal projections for VOXZOGO revenues.
Rest of World: Primarily Latin America, Middle East, and Asia.

Plans for Strong Growth in the U.S. with VOXZOGO in Achondroplasia

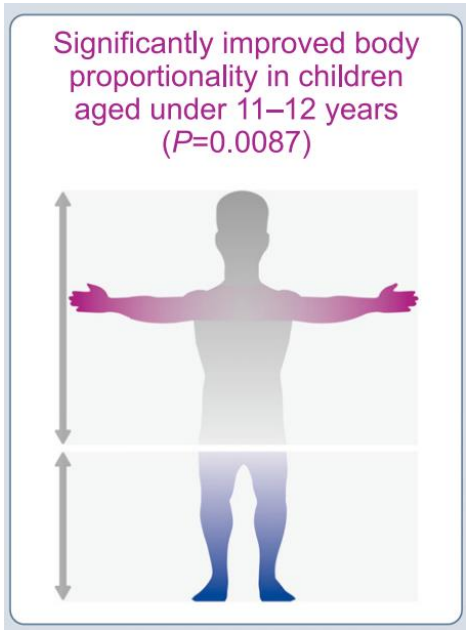


VOXZOGO U.S. Strategy

- Expected to be the only approved product for achondroplasia, from birth, through 2027
- Majority of new patients are in 0-5 age group since label expansion in 2023
- Continued expansion of U.S. prescriber base, especially pediatric endocrinologists
- Strong progress raising awareness to drive referrals from pediatricians
- New treatment guidelines for VOXZOGO and growing body of clinical benefit data beyond height

New Publications Highlight Importance of Early VOXZOGO Treatment

VOXZOGO Treated Children Demonstrated Statistically Significantly Improvements in Proportionality vs. Untreated Control Arm



VOXZOGO phase 3 treatment in achondroplasia (n=48)

Published in *Med,* December 2024[1]

New International Treatment Guidelines Recommend Early Diagnosis and Treatment with VOXZOGO

New International Treatment Guidelines:

1. **Early screening & detection of achondroplasia** (prenatally, where possible)
2. **Education & consent for VOXZOGO treatment**
3. **Begin VOXZOGO treatment as early as possible**
4. **Monitor treatment outcome and response after VOXZOGO initiation**
5. **Ongoing monitoring until growth plate closure**

VOX label:
U.S.: from birth
EU: 4ms+

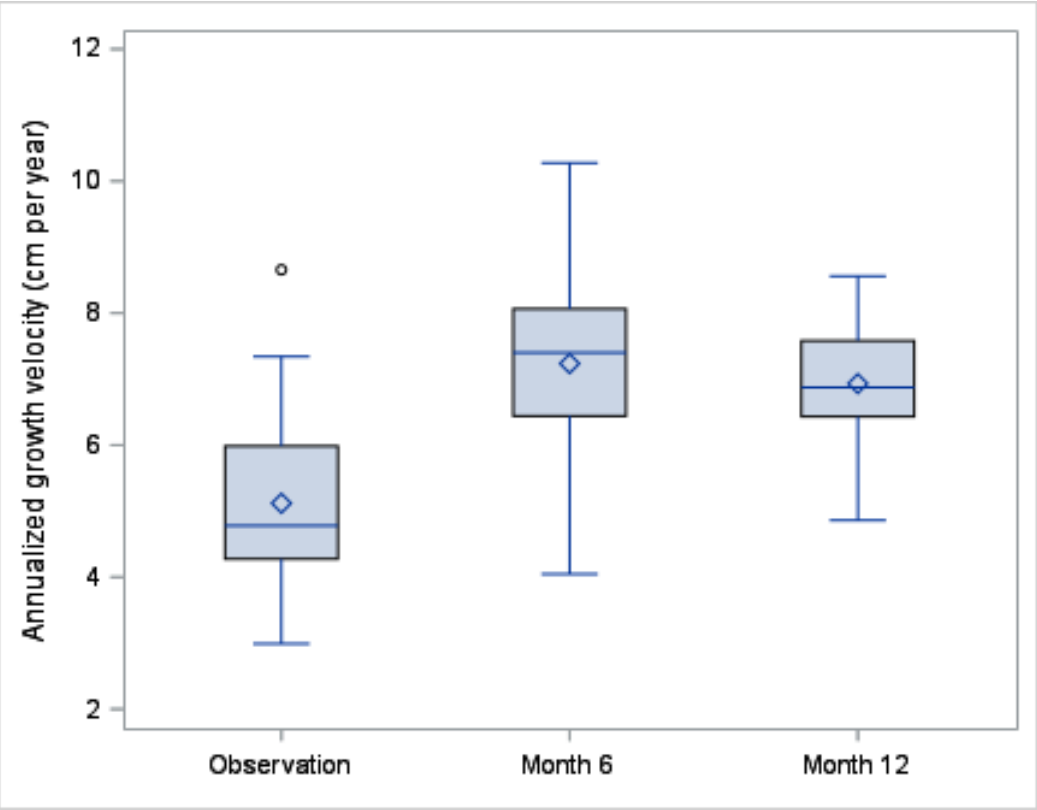
Published in *Nature Reviews Endocrinology,* January 2025[2]

[1]Savarirayan et al., Sustained growth-promoting effects of vosoritide in children with achondroplasia from an ongoing phase 3 extension study, Med (2024), https://doi.org/10.1016/j.medj.2024.11.019
[2]Savarirayan, R., Hoover-Fong, J., Ozono, K. *et al.* International consensus guidelines on the implementation and monitoring of vosoritide therapy in individuals with achondroplasia. *Nat Rev Endocrinol* (2025). https://doi.org/10.1038/s41574-024-01074-9

VOXZOGO for Hypochondroplasia (HCH) Demonstrated a 1.81cm Increase in Annualized Growth Velocity in Phase 2

Potential First-in-class Approved Treatment Option; Tracking to Expected 2027 Launch

Annualized Growth Velocity



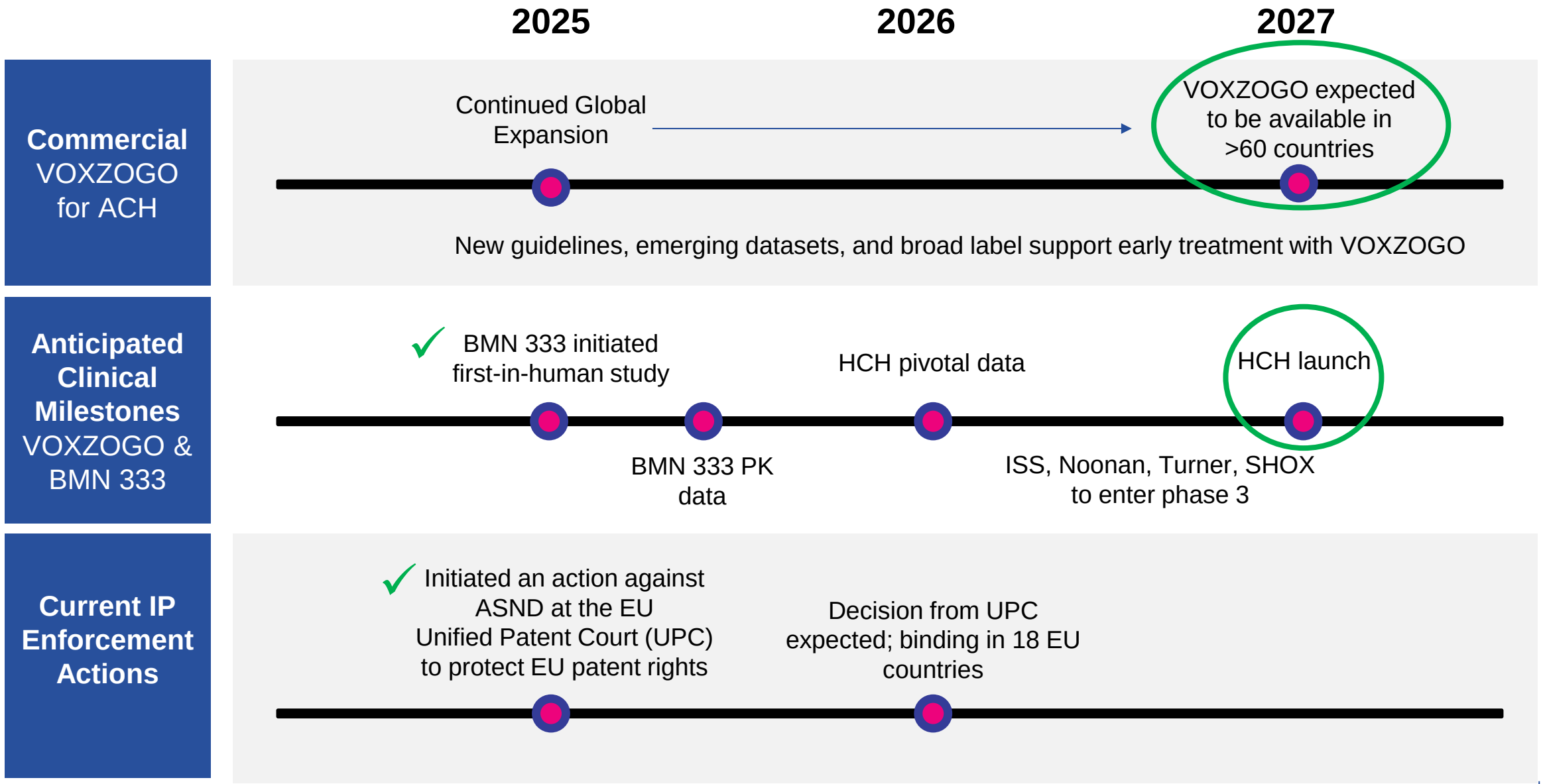
Phase 2 data[1]: N = 24, 1.81 cm/year increase in mean AGV

Clearly Defined Go-to-market Strategy

- Rapid recruitment of pivotal study, expected to be fully enrolled in 1Q'25
- Direct-to-registrational opportunity enabled with evidence from achondroplasia and early proof-of-concept in HCH
- Targeting ~14,000 most severely impacted HCH patients (those with height Z-score ≤ -2.0 SDs)
- Leveraging 5-year global achondroplasia leadership
- Focus on increasing diagnosis and establishing treatment paradigm

[1]Vosoritide treatment for children with hypochondroplasia: a phase 2 trial. Dauber, Andrew et al. eClinicalMedicine, Volume 71, 102591

Building Leadership in Skeletal Conditions



Underlying, Established Strengths of the Enzyme Therapies Business

~$1.4B Enzyme Therapies Revenues 3Q'24 Year-to-date

High Patient Reach and Treatment Continuity

First-in-category Medicines

Challenging Path for Biosimilars

- Built and created global leadership in each therapeutic area
- 20-year track record building Enzyme Therapies infrastructure foundational to sustained growth
- Profile of these transformational medicines support high compliance and adherence rates

VIMIZIM™ (elosulfase alfa)

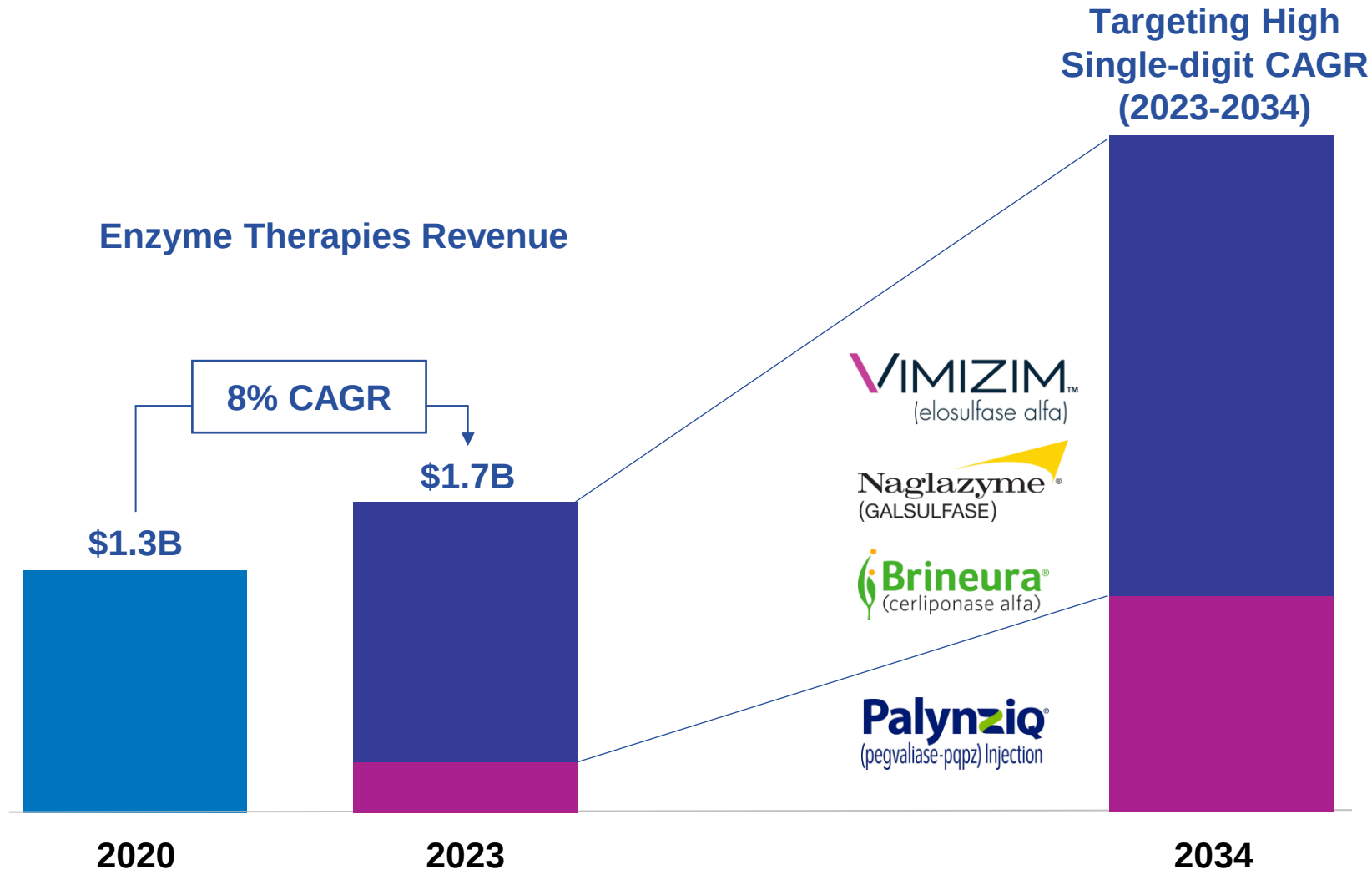
Naglazyme® (GALSULFASE)

Brineura® (cerliponase alfa)

Palynziq® (pegvaliase-pqpz) Injection

ALDURAZYME® (LARONIDASE)

Strategy to Drive Long-Term Growth across Enzyme Therapies Business



Find & Start New Patients

- Expand access to diagnostic tools, including gene panels
- Implement initiatives to increase diagnosis, including cascade screening
- Leverage predictive analytics to pinpoint hot spots

Grow PKU Opportunity

- Expand label to include 12-17 y/o
- Broaden geographic reach
- Develop BMN 390: Next-gen Palynziq with potential for lower immunogenicity

Note: Revenue figures include revenues for Vimizim, Naglazyme, Brineura, Palynziq, and Aldurazyme. 2034 revenues include expected revenues for BMN 390 for phenylketonuria.

External Innovation Strategy to Complement Revenue Growth

Leadership in Development & Commercialization of Medicines in Genetically Defined Conditions

Rich Environment for Business Development

- Scientific momentum allows clearly defined drug development
- Rich biotech arena with many small companies at R&D stage
- Biotech remains capital constrained

BioMarin as the Partner of Choice

- Proven world class research, clinical, and regulatory capabilities
- Best-in-class manufacturing
- Strong global commercial infrastructure

Focus on Disciplined Deal Execution < $1.5B

- Deals ranging from pre-clinical to clinical stage
- New internal world-class Business Development capabilities and experience
- Focus on Skeletal Conditions, Enzyme Therapies, First in Genetic Conditions

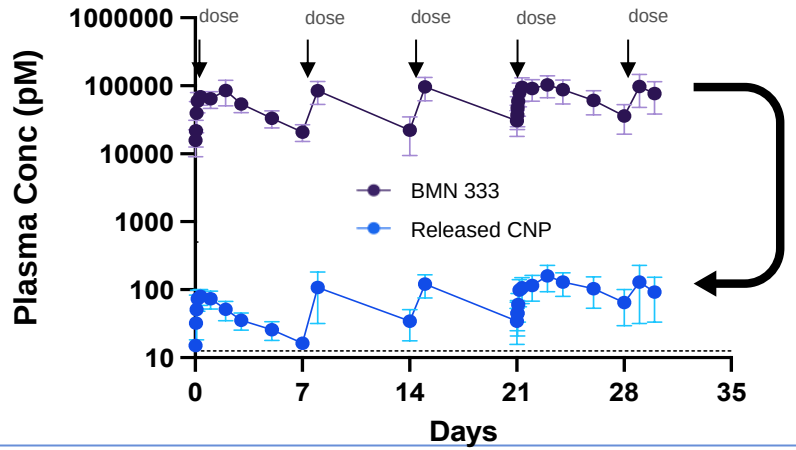
BMN 333: Opportunity to Optimize PK and Clinical Profile in ACH

BMN 333 – Longer-Acting CNP

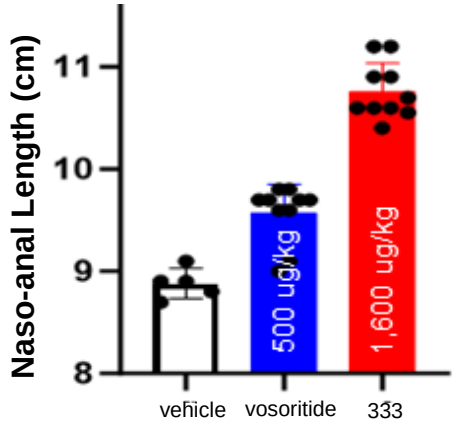
- **Half-life prolongation** chemistry similar to other approved drugs (weekly dosing)
- **Free-CNP exposure** predicted to surpass all published in-class (multi fold-level)
  - animal models suggest meaningful additional growth is possible
- **Experience** with VOXZOGO provides clear PK targets for free-CNP and clinical targets for PD

Phase 1 PK 2025 → **Target Dose Ranging Start: 2026** → **Target Pivotal Start: TBD** → **Target Launch 2030**

SQ dosing of BMN 333 releases CNP in NHPs

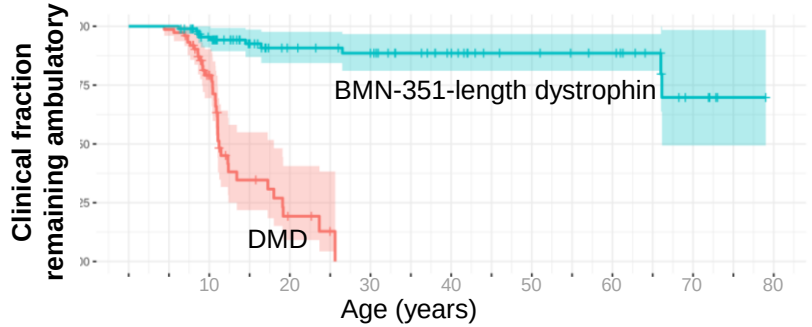


BMN 333 Increases Skeletal Growth to a greater extent than high doses of pulsatile CNP in mice



BMN 351 for Duchenne Muscular Dystrophy; Targeting 2025 POC

- **Oligonucleotide for Exon-51 Duchenne Muscular Dystrophy**
- **Novel splice site** for exon-51 skip induction, targeting >10% dystrophin levels at steady state
- **Near Full-Length Dystrophin** produced, naturally-occurring versions of which preserve ambulation into adulthood



- **Phosphorothioate chemistry** (non-morpholino) leverages in-house expertise

Phase 1/2 2024 → **Target POC 2025** → **Target Ph3 Start: 2026** → Target Launch **2028**

Preclinical Evidence Published

1. Muscle cell penetration	✓ WMS 2020
2. Binding at novel splice site with skip-induction	✓ FUTURES 2022 ✓ *In vitro* TIDES EU 2023
3. Functional dystrophin expression	✓ WMS 2020 ✓ TIDES USA 2020
4. Functional benefit	✓ WMS 2023 ✓ SfN 2023

Clinical Evidence

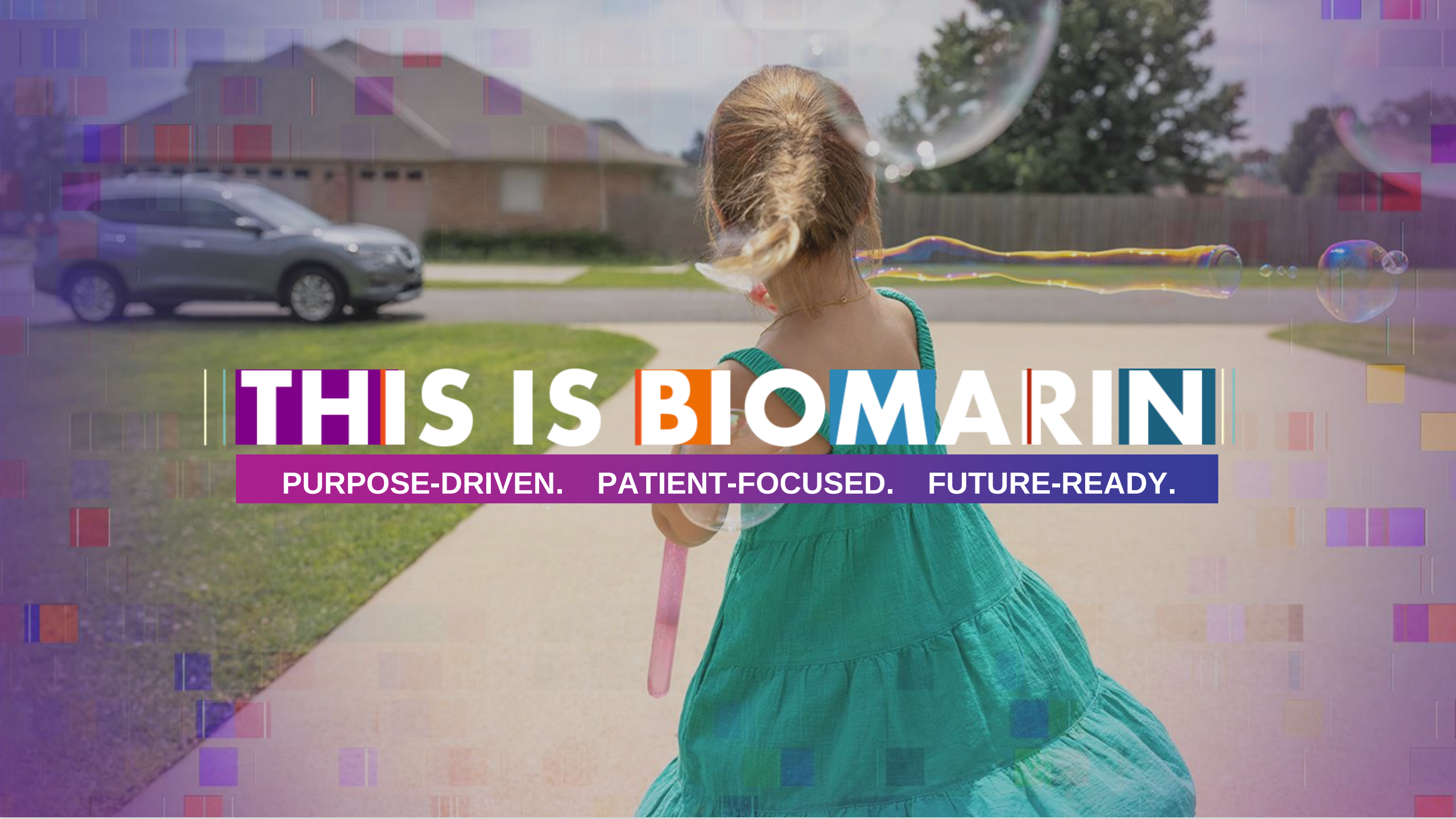
1. Muscle cell penetration	✓
2. Binding at novel splice site with skip-induction	✓ **Interim 13-wk data**
3. Functional dystrophin expression	✓

❑ **Anticipate release of 26-week data in 2H'25**

WMS = World Muscle Society Annual Congress; **FUTURES** = CureDuchenne National Conference; **TIDES** = Oligonucleotide and Peptide Therapeutics Conference; **SfN** = Society for Neuroscience Annual Meeting

Anticipated Near-Term R&D Milestones

	Candidate	Condition	18-Month Look-Ahead
Skeletal Conditions	VOXZOGO	Hypochondroplasia	**1H25:** Pivotal trial fully enrolled
	VOXZOGO	Idiopathic Short Stature	**1H25:** Phase 2 study enrolling
	VOXZOGO	Noonan/Turner/SHOX Syndrome	**1H25:** Phase 2 study enrolling
	BMN 333	ACH & HCH	**2H25:** PK data; **1H26:** PK presentation
Enzyme Therapies	PALYNZIQ	Phenylketonuria (12-17)	**1H25**: Phase 3; **2H25:** sBLA filing
	BMN 390	Phenylketonuria (Novel PEG)	**1H26:** FIH study start
Pipeline	BMN 351	Duchenne Muscular Dystrophy	**2H25:** Clinical POC presentation
	BMN 349	Alpha-1 Antitrypsin Deficiency	**1H25:** Patient study start
	BMN 370	von Willebrand Disease	**2026:** FIH study start


THIS IS BIOMARIN
PURPOSE-DRIVEN. PATIENT-FOCUSED. FUTURE-READY.

Thank you.

Q&A

Appendix

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information[1]

	Nine Months Ended September 30,	
	2024	2023
GAAP Reported Net Income	**$ 302**	**$ 147**
Adjustments		
Stock-based compensation expense - COS	12	13
Stock-based compensation expense - R&D	45	49
Stock-based compensation expense - SG&A	93	90
Amortization of intangible assets	34	47
Gain on sale of nonfinancial assets [2]	(10)	—
Severance and restructuring costs [3]	86	(1)
Loss on investments [4]	5	13
Income tax effect of adjustments	(61)	(48)
Non-GAAP Income	$ 506	$ 310

(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 a downward adjustment to non-marketable equity securities recorded in Other income (expense), net.

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information[(1)]

	Nine Months Ended September 30,			
	2024	Percent of GAAP Total Revenue	2023	Percent of GAAP Total Revenue
GAAP Income from Operations	**$ 323**	**15.3 %**	**$ 158**	**8.9 %**
Adjustments				
Stock-based compensation expense	150	7.2	152	8.6
Amortization of intangible assets	34	1.6	47	2.7
Gain on sale of nonfinancial assets [(2)]	(10)	(0.5)	—	—
Severance and restructuring costs [(3)]	87	4.1	(1)	(0.1)
Non-GAAP Income from Operations	$ 583	27.7 %	$ 357	20.1 %

(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.

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information[1]

	Nine Months Ended September 30,	
	2024	2023
GAAP Diluted EPS	**$ 1.56**	**$ 0.77**
Adjustments		
Stock-based compensation expense	0.76	0.76
Amortization of intangible assets	0.17	0.24
Gain on sale of nonfinancial assets [2]	(0.05)	—
Severance and restructuring costs [3]	0.44	—
Loss on investments [4]	0.03	0.06
Income tax effect of adjustments	(0.31)	(0.23)
Non-GAAP Diluted EPS	$ 2.60	$ 1.60

(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 a downward adjustment to non-marketable equity securities recorded in Other income (expense), net.

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information(1)

	Nine Months Ended September 30,	
	2024	2023
GAAP Weighted-Average Diluted Shares Outstanding	**196.7**	**195.0**
Adjustments		
Common stock issuable under the company's convertible debt (1)	—	4.4
Non-GAAP Weighted-Average Diluted Shares Outstanding	196.7	199.4

(1) Common stock issuable under the company's convertible debt was excluded from the computation of GAAP Weighted-Average Diluted Shares Outstanding when they were anti-dilutive. If converted, for the prior year comparative period, the company would have issued approximately 4.4 million shares under the convertible notes due in 2027.