# Welcome

## Investor Day

September 4, 2024

# 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, in certain instances, the full-year 2024 and future periods and the underlying assumptions; BioMarin's new corporate strategy, including plans and expectations regarding innovation and growth, the $500 million cost optimization program, capital allocation, and organizational redesign efforts, as well as the anticipated benefits of such strategy, including BioMarin's ability to achieve top quartile biopharma revenue growth, double its Non-GAAP Operating Margin, achieve innovation and efficiencies, optimize cash flow and capital allocation, and deliver significant and sustained value creation to stakeholders; BioMarin's future strategy for ROCTAVIAN and its anticipated benefits, including BioMarin's expectations regarding reduction of annual direct ROCTAVIAN expenses beginning in 2025 and ROCTAVIAN being profitable by the end of 2025; 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; and 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.

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 September 4, 2024 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, 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 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.

# Market, Industry and Third-Party Data and Other Notices

## Market, Industry and Third-Party Data

This presentation contains estimates, projections and other information concerning BioMarin's industry, business and the potential markets for BioMarin's commercial products and product candidates (if approved), including data regarding the estimated size of such markets and the incidence of certain medical conditions. BioMarin obtained the industry, market and similar data set forth in this presentation from its internal estimates and research and from academic and industry research, publications, surveys and studies conducted by third parties, including governmental agencies.

While BioMarin believes that the data it uses from third parties is reliable, BioMarin has not separately verified these data and cannot assure you of the data's accuracy or completeness. This information, to the extent it contains estimates or projections, involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates or projections. The industry in which BioMarin operates is subject to risks and uncertainties due to a variety of factors, which could cause results to differ materially from those expressed in these publications and reports.

## Other Notices

This presentation is not intended to promote the products referenced herein or otherwise influence healthcare prescribing decisions and does not constitute an offer to sell, or the solicitation of an offer to buy, any securities or a solicitation of any vote or approval. BioMarin®, BRINEURA®, KUVAN®, NAGLAZYME®, PALYNZIQ®, ROCTAVIAN®, VIMIZIM® and VOXZOGO® are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. ALDURAZYME® is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this release are the property of their respective owners.

# Introduction:

# BioMarin's New Corporate Strategy to Deliver Significant and Sustained Value Creation to Stakeholders

**ALEXANDER HARDY**
President & Chief Executive Officer

# BioMarin's Leadership Team

**Alexander Hardy**

President and Chief Executive Officer

**Henry Fuchs, M.D.**

President, Worldwide Research & Development

**Eric Davis**

Executive Vice President, Chief Legal Officer

**Greg Guyer, Ph.D.**

Executive Vice President, Chief Technical Officer

**Cristin Hubbard**

Executive Vice President, Chief Commercial Officer

**Marni Kottle**

Executive Vice President, Chief Corporate Affairs Officer

**Brian Mueller**

Executive Vice President, Chief Financial Officer

**Amy Wireman**

Executive Vice President, Chief People Officer

**Greg Friberg, M.D.**

Executive Vice President, Chief Research & Development Officer

*BioMarin Start Date: September 30, 2024*

**James Sabry, M.D., Ph.D.**

Executive Vice President, Chief Business Officer

*BioMarin Start Date: October 7, 2024*

# Today's Agenda

| | |
|---|---|
| **Chapter 1: Welcome**<br>BioMarin's New Corporate Strategy to Deliver Significant and Sustained Value Creation to Stakeholders | **Alexander Hardy** – President and Chief Executive Officer |
| **Chapter 2: Value Commitment**<br>Financial Strategy to Deliver High Growth and Superior Returns | **Brian Mueller** - Executive Vice President, Chief Financial Officer |
| **Chapter 3: Innovation**<br>Innovation Strategy to Deliver Sustainable Pipeline of High Impact Medicines | **Hank Fuchs, MD** – President, Worldwide Research & Development |
| **Chapter 4: Growth**<br>Optimizing BioMarin's Growing and Durable Enzyme Therapies Business Unit<br><br>Building Leadership in Achondroplasia To Set the Stage for Multiple New Indications | **Cristin Hubbard** – Executive Vice President, Chief Commercial Officer |
| Q&A | **BioMarin Executive Team** |

# Progress on Strategic Priorities since January

**ACCELERATE AND MAXIMIZE THE VOXZOGO OPPORTUNITY**

- Robust VOXZOGO revenue growth (67% in 1H'24 Y/Y)
- Five new indications accelerated, including FDA alignment
- Resolved VOXZOGO supply constraint ahead of plan

**ESTABLISH ROCTAVIAN OPPORTUNITY**

- Commitment to achieve profitability by YE25 by reducing ROCTAVIAN expenses to ~$60M
- Focus on United States, Germany and Italy
- Increased focus on site-level market access

**FOCUS R&D ON THE MOST PROMISING ASSETS**

- Evaluated portfolio using stringent criteria including patient impact and shareholder value
- Prioritized three programs and identified opportunities to accelerate
- Discontinued five programs, resulting in planned net reductions to operating expenses

**ACCELERATE EPS GROWTH AND EXPAND MARGINS**

- Expect 15% total revenue growth in 2024 (guidance midpoint)
- Delivered 1H'24 EPS growth significantly higher than revenue growth
- Implemented corporate reorganization and optimization to drive substantial efficiency

# BioMarin Today

**25-year**

legacy of innovation

**8**

commercialized products

**6**

first-in-disease products

**~90%**

track record of regulatory approvals post Phase 2* in the last 20 years

**~80**

countries within our global commercial footprint

**$2.75B to $2.825B**

FY24 revenue guidance

**2023 Actuals** **2024 Guidance**

**19% → 26-27%**

Full Year Non-GAAP Operating Margin**

**$1.8B**

cash and investments on hand at 2Q24***

*Post-Phase 2 studies conducted by BioMarin

**Refer to slide 3 and the appendix to this presentation for the definition of Non-GAAP Operating Margin and reconciliations to the comparable information reported under U.S. GAAP with respect to historical Non-GAAP Operating Margin. Reconciliation of forward-looking Non-GAAP Operating Margin to the most directly comparable U.S. GAAP reported financial measure is not available. Refer to the appendix slide 78 for further information regarding forward-looking Non-GAAP financial measures.

***$1.8B includes cash, cash equivalents, and investments; ~$500M convertible debt paid in August 2024, settled in cash

# OUR PURPOSE

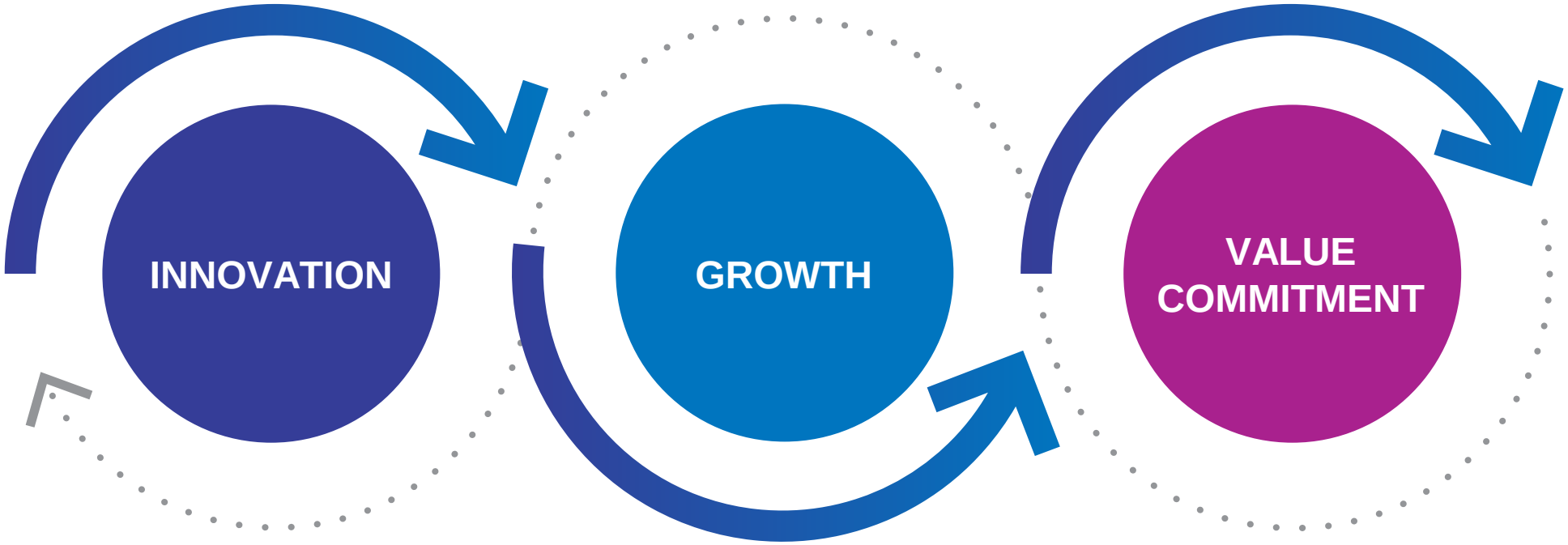
Be the biotech leader that translates the promise of genetic discovery into medicines that make a profound impact on the life of each patient.




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

By 2034, we hope to have **~4X** as many patients on therapy as we serve today

# Our Strategy to Deliver Significant Value Creation



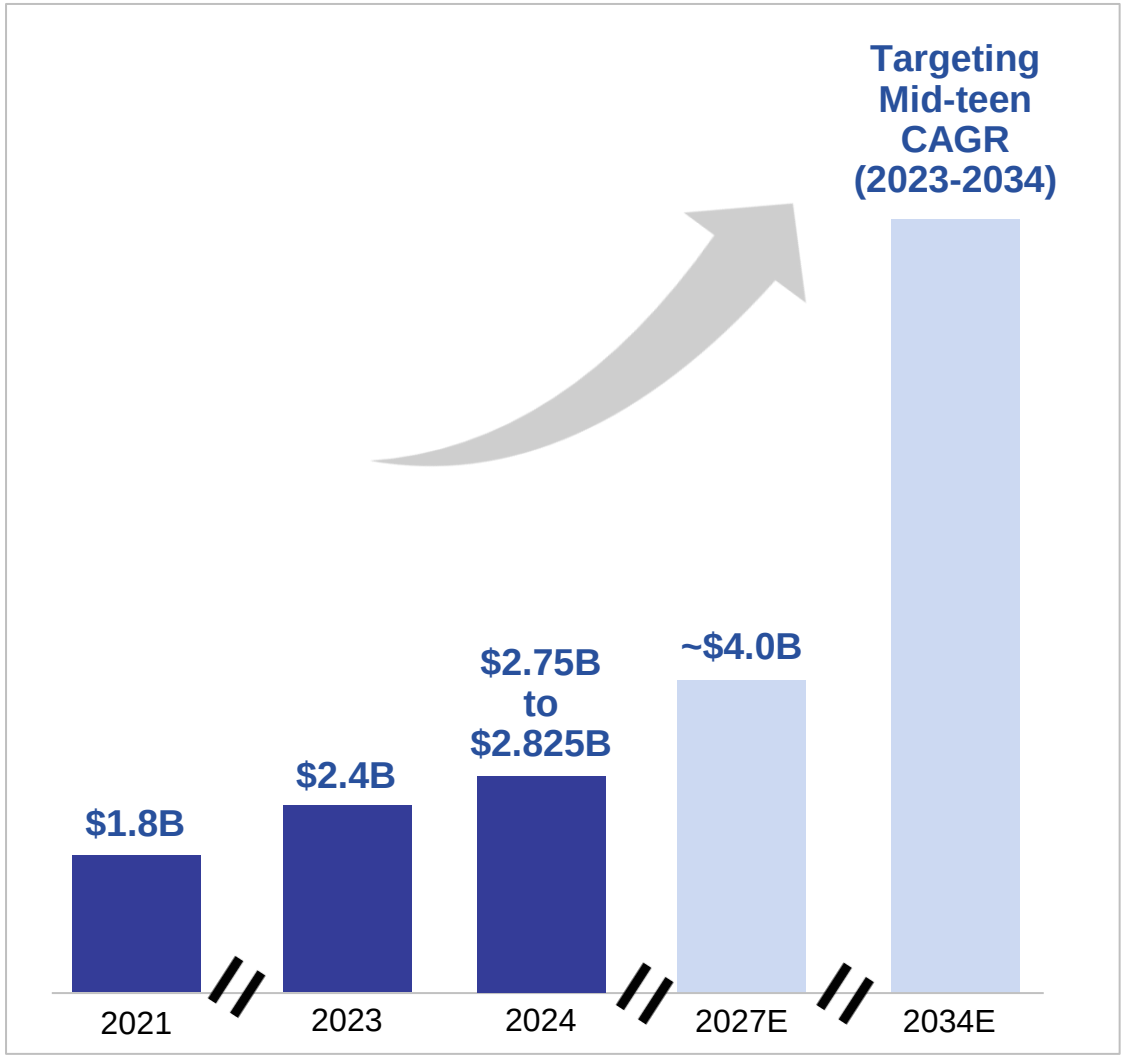

- Differentiated innovation engine
- Prioritized R&D pipeline
- Sustainability driven by genomics revolution

- Enzyme Therapies revitalized growth strategy
- VOXZOGO as sustainable growth driver in achondroplasia alone
- 5 new VOXZOGO indications advancing

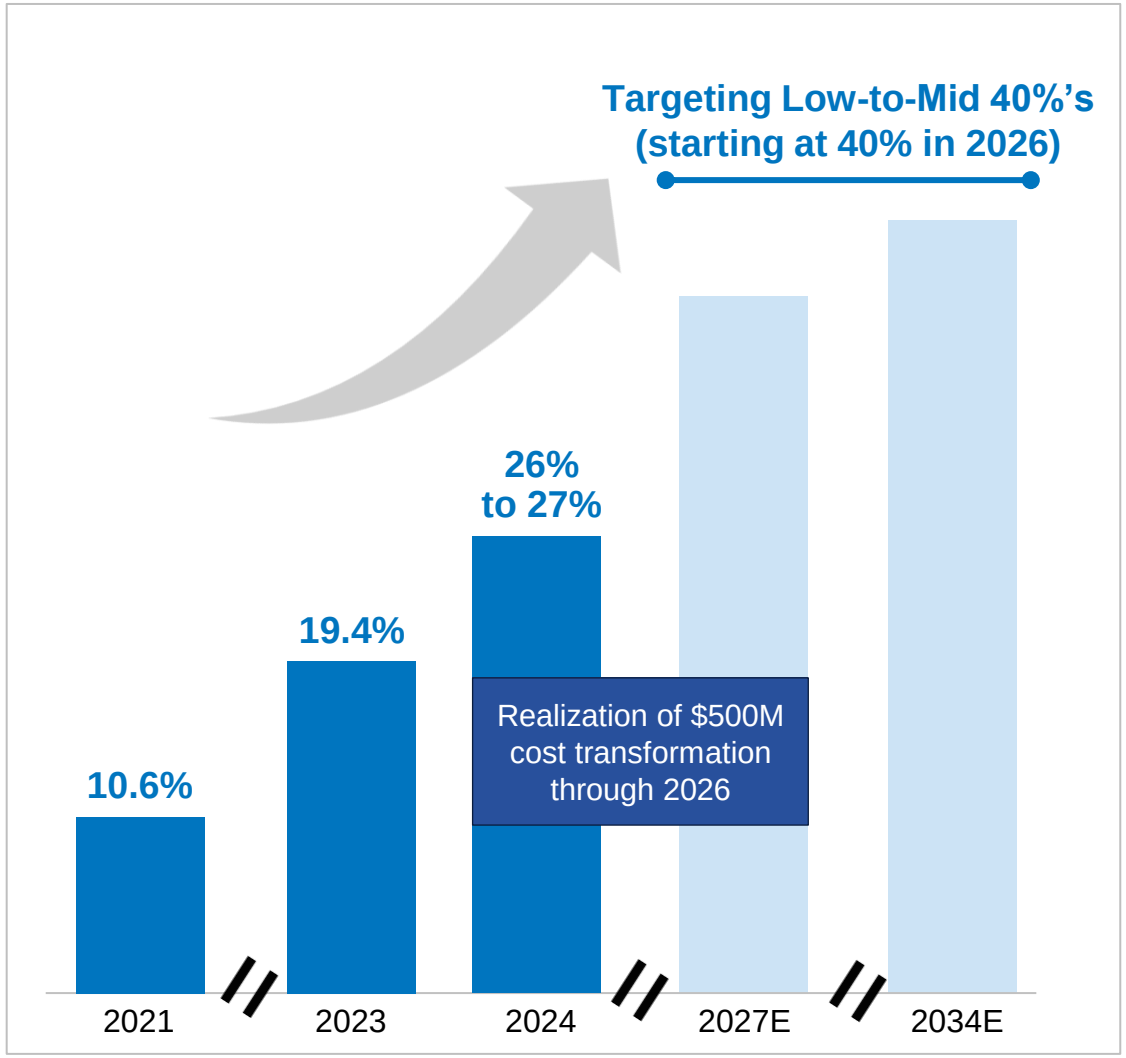
- Accelerating profitability
- Increasing operating cash flow
- Business development to augment growth

# Our Value Commitment: Sustained Growth & Margin Expansion

## Total Revenues




## Non-GAAP Operating Margin*

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# New Business Units to Drive Accountability & Improve Efficiency

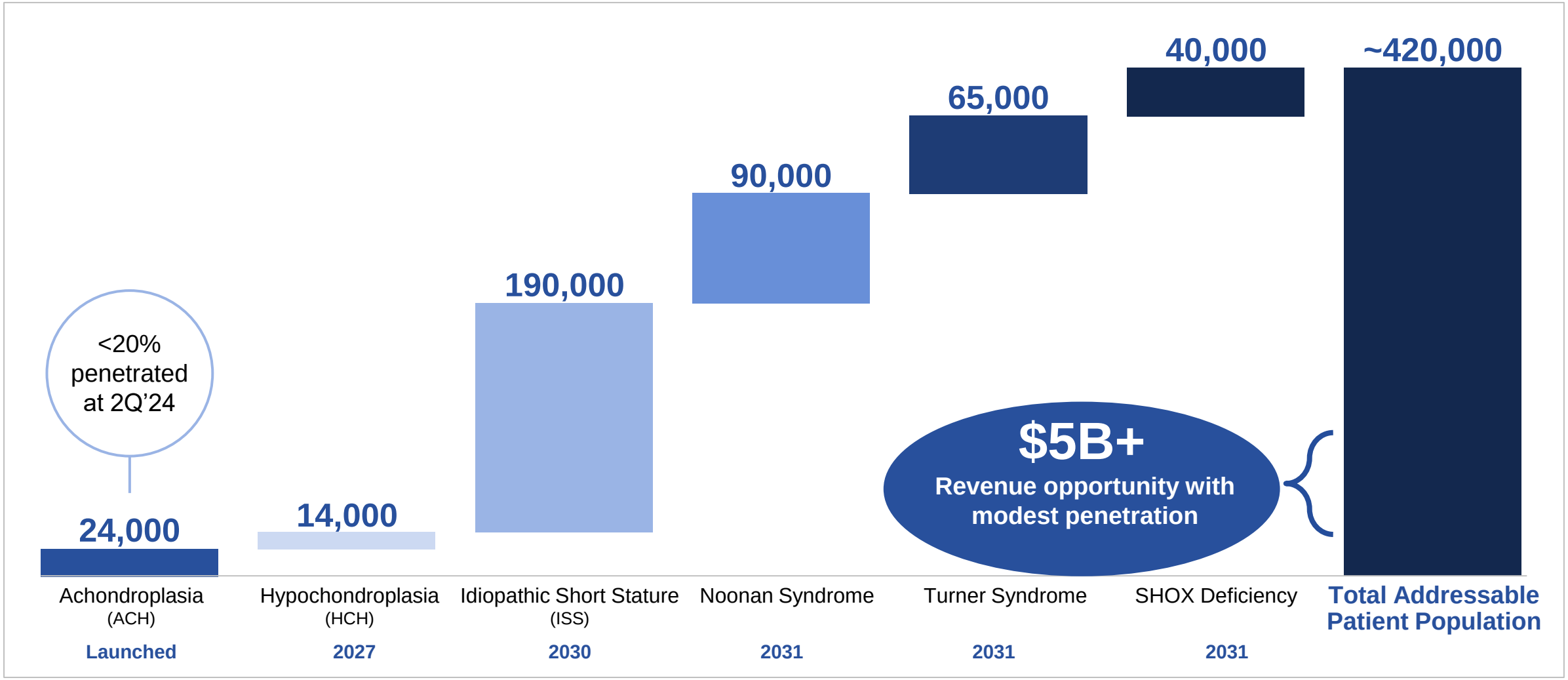

- Recognizes different strategic contexts of these businesses
- Dedicated leadership with authority, accountability and a sense of urgency
- Optimized and transparent resource allocation focused on executing against top priorities
- Targeted top and bottom-line objectives by unit
- Facilitated by enterprise-wide operating model and cultural evolution

Refer to appendix slide 71 for programs included in respective business units

**Skeletal Conditions**

# Leveraging Achondroplasia to Build a $5B+ Franchise

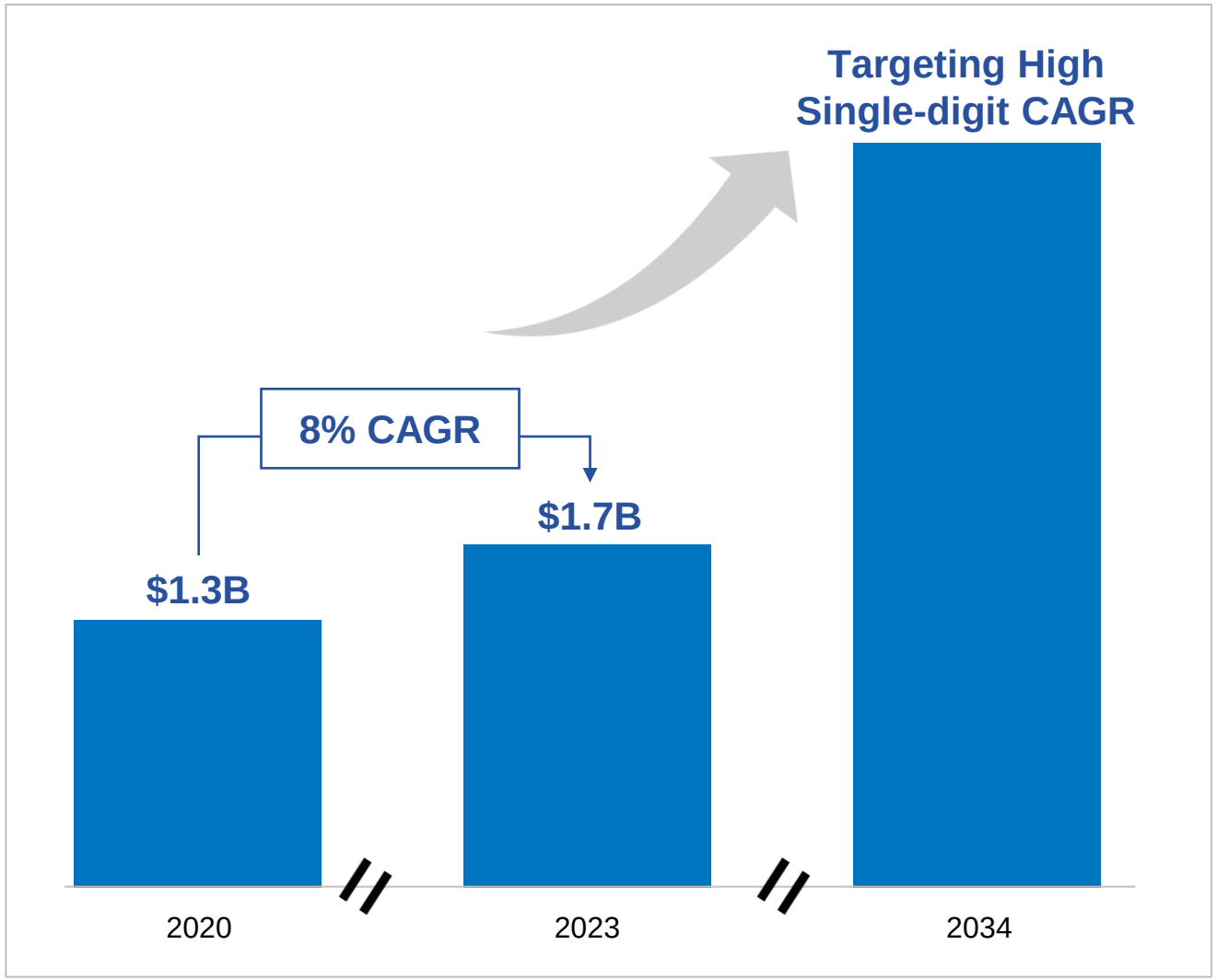
## Total Addressable Patient Population (TAPP)*




*TAPP defined as the diagnosed, clinically eligible patients for a given product or program in a defined footprint; footprint for above indications (except ISS) defined as all markets included in BioMarin's internal projections for VOXZOGO revenues. ISS footprint is US-only. Height Z-score eligibility for TAPP: ACH (N/A); HCH (≤ -2.0 standard deviations (SDs)); ISS (≤ -2.5 SDs); Noonan Syndrome (≤ -2.5 SDs); Turner Syndrome (≤ -2.5 SDs); SHOX Deficiency (≤ -2.5 SDs)

# Growing Durable Market-leading Franchise

## Enzyme Therapies Revenue




VIMIZIM™ (elosulfase alfa)

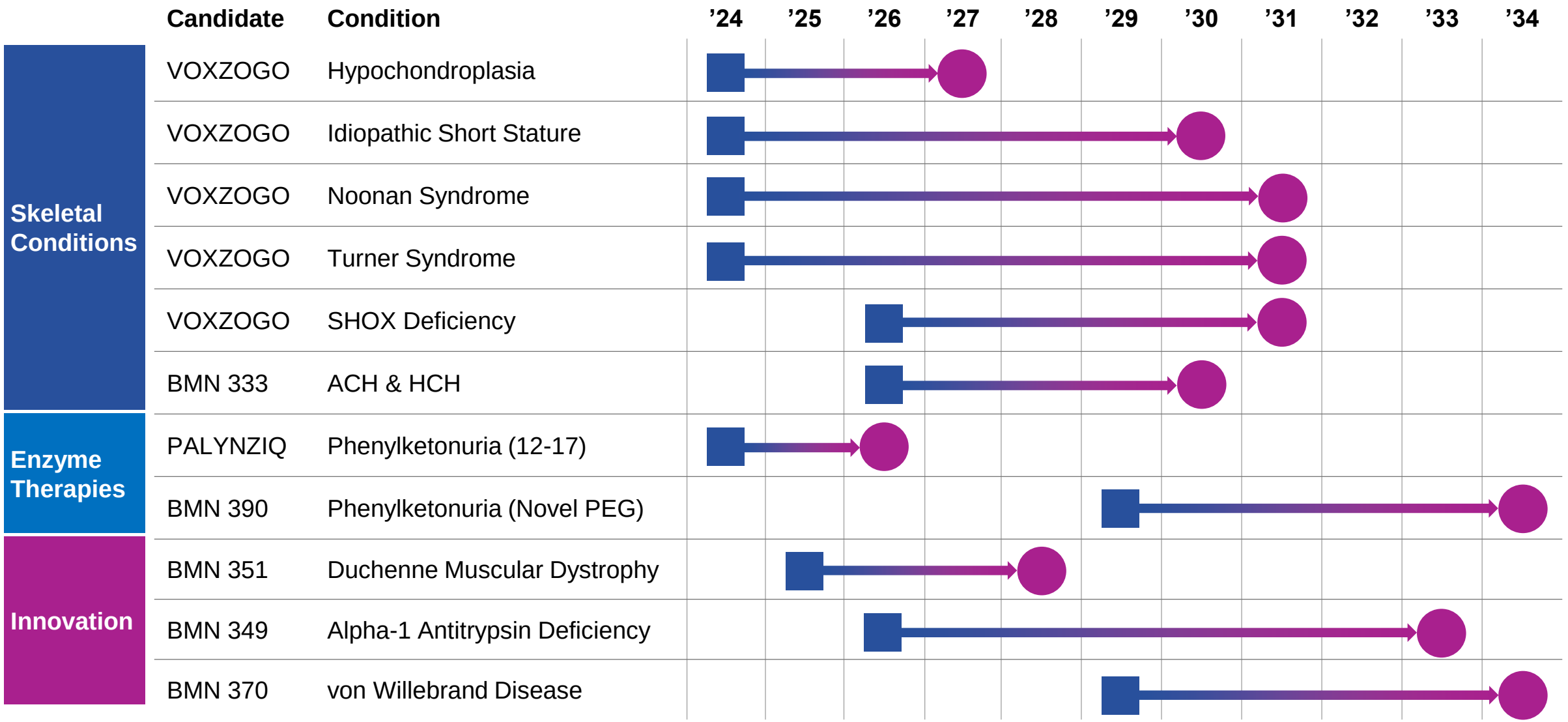
Naglazyme® (GALSULFASE)

Palynziq® (pegvaliase-pqpz) Injection

Brineura® (cerliponase alfa)

ALDURAZYME® (LARONIDASE)

# Targeting 11 Launches by 2034, Two Launches by 2027



# Expected Organic Growth Can Be Augmented with External Assets

## Target-rich Environment

- Leverage the genomic revolution
- Scientific momentum continues at academia and start ups
- Early-stage biotech remains capital constrained

## Right to Win

- BioMarin's Core 5
- Proven world-class clinical, regulatory, and manufacturing capabilities
- Best-in-class global genetic disease commercialization

## Focused on Deal Execution <$1.5B

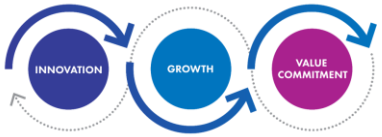
- ~$1B+* in deployable cash & generating increasing deployable capital and leverage capacity
- BioMarin as the partner of choice
- New internal world-class Business Development capabilities and experience

**Responsible & Disciplined Deployment of Growth Capital within Specific Focus Areas**

**Skeletal Conditions | Enzyme Therapies | First in Genetic Disease (Development & Commercial)**

*$1.8B includes cash, cash equivalents, and investments; ~$500M convertible debt paid in August 2024, settled in cash

# A Compelling Growth Strategy




| Top Quartile Biopharma Revenue Growth* | Doubling Non-GAAP Operating Margin** | Prioritized Innovation | Optimized Cash Flow & Capital Allocation |
|---|---|---|---|
| **TARGETING:** | **TARGETING:** | **TARGETING:** | **TARGETING:** |
| $4B revenue in 2027<br>Mid-teen Revenue Growth through 2034 | 40%+ Non-GAAP Operating Margin Starting in 2026 | 7 Indications in Phase 3 by 2027<br>11 Launches by 2034, including Two by 2027 | $1.25B+ Operating Cash Flow In 2027 and Beyond |

*Refer to appendix slide 70 for BioMarin peer comparisons for top quartile revenue growth
**Refer to slide 3 and the appendix to this presentation for the definition of Non-GAAP Operating Margin. Reconciliation of forward-looking Non-GAAP Operating Margin to the most directly comparable U.S. GAAP reported financial measure is not available. Refer to the appendix slide 78 for further information regarding forward-looking Non-GAAP financial measures.

# Value Commitment:

# Financial Strategy to Deliver High Growth and Superior Returns

**BRIAN MUELLER**
Executive Vice President,
Chief Financial Officer

# Long-term Guidance is in Top Quartile

| | 2023 | 2024 | 2027 | 2034 |
|---|---|---|---|---|
| REVENUE | $2.4B | $2.750B to $2.825B | $4B<br>**Skeletal Conditions** >25% CAGR*<br>**Enzyme Therapies** high single-digit CAGR* | Targeting **Mid-teen** CAGR |
| NON-GAAP OPERATING MARGIN** | 19.4% | 26% - 27% | **Low-to-Mid 40%'s**, starting with **40%** in **2026** | |
| OP CASH FLOW | $0.2B | | $1.25B+ Operating Cash Flow | |

*Compounded annual growth rate 2023 through 2027

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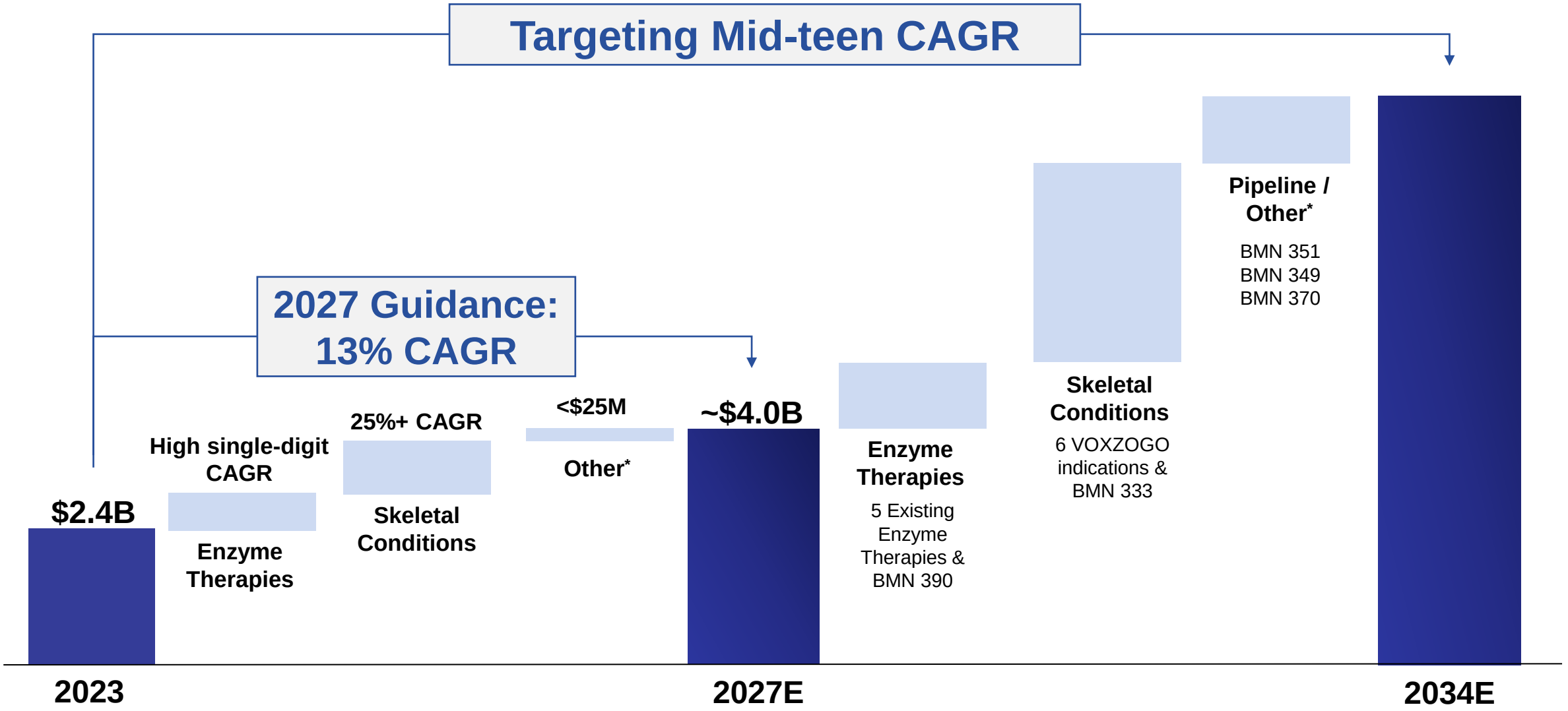
# Strategy Drives Mid-teen Revenue CAGR Through 2034




**Non-risk-adjusted: Targeting Mid-teen CAGR ('23-'34)**
**Risk-adjusted: Targeting ~10% CAGR ('23-'34)**

*Other includes ROCTAVIAN, Kuvan, and Royalty Revenue
All future revenue shown on a non-risk-adjusted basis and excludes future potential external assets

# Implementation of $500M Cost Optimization Program

**2023** — **Fully Realized Savings Timeline** — **2026**

## Prioritized R&D

*Continual Scrutiny of Program Scientific and Commercial Prospects*

## Operating Model Efficiencies

*Enterprise-wide Reorganization with Business Units and Streamlined G&A*

## Focused ROCTAVIAN Investment

*Spend Rebalancing to Support Launch Momentum with Upside Potential*

## COGS Efficiency

*Ongoing Manufacturing Efficiencies*

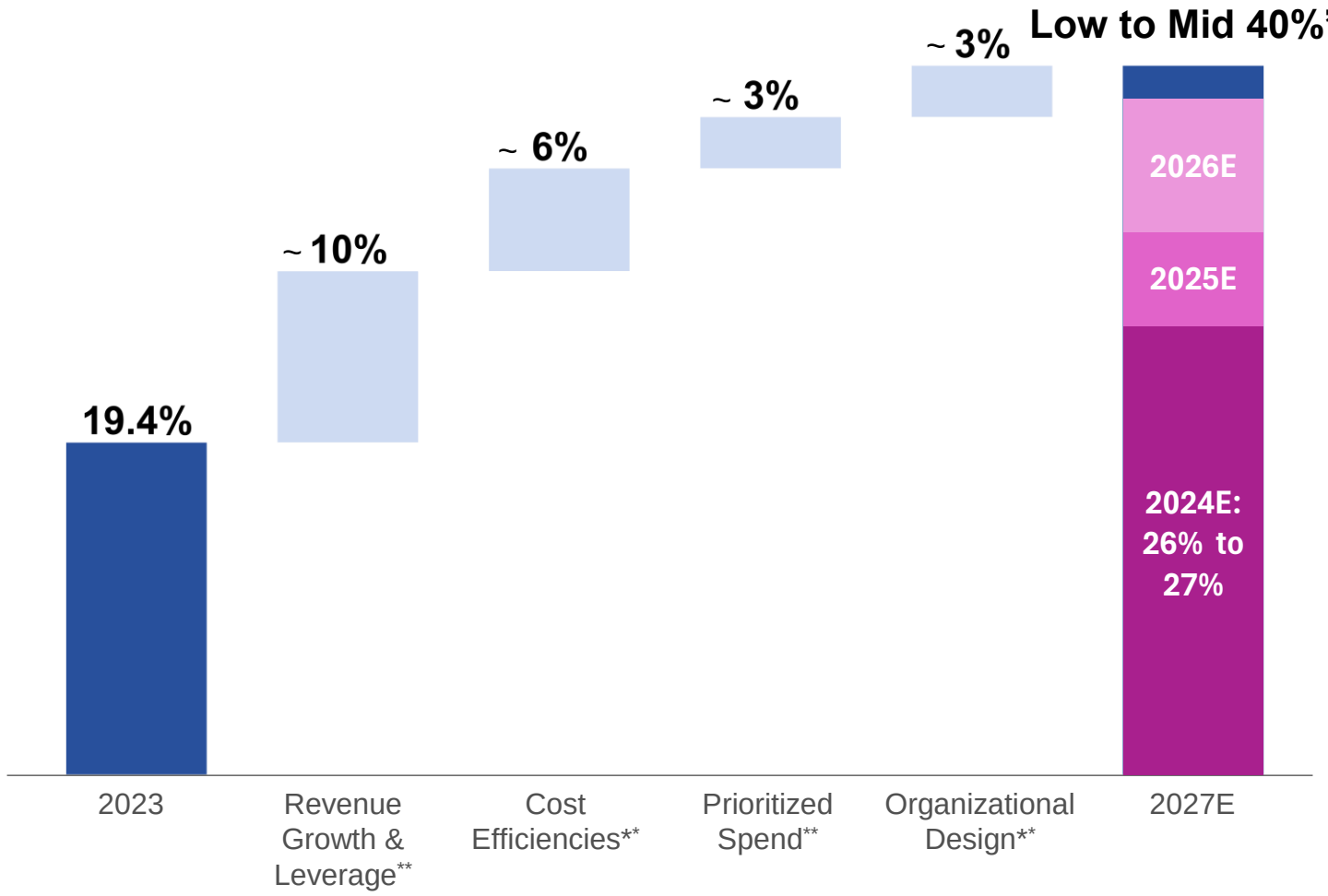
## Sourcing & Procurement

*External Spend Optimization*

## Global Business Services

*Global Process Consolidation into a Lower Cost Location*

# Targeting to Double Non-GAAP Operating Margin*




| P&L Line | 2023 Actuals | 2027 Expectation |
|---|---|---|
| Gross Margin % | 78.7% | 82.5% - 85.0% |
| R&D % | 28.2% | 21.0% - 24.0% |
| SG&A % | 31.2% | 19.5% - 22.5% |
| Non-GAAP Operating Margin %* | 19.4% | Low to Mid 40%'s |

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**Revenue growth includes investments to support revenue growth. Cost efficiencies includes manufacturing and procurement, amongst others. Prioritized Spend Includes discontinued programs, ROCTAVIAN, and prioritized R&D investments. Organizational redesign includes new operating model and Global Business Services.

# Our Capital Allocation Priority is Investing in Future Growth

## DISCIPLINED DEPLOYMENT OF INCREASING FREE CASH FLOW ALIGNED WITH STRATEGY

| BALANCE SHEET HIGHLIGHTS |
| --- |
| **$1.8B**<br>Cash and investments as of June 30, 2024* |
| **$1.25B+**<br>Annual Operating Cash Flow by 2027 |
| **$600M**<br>Credit facility (new in 3Q24) |

**CAPITAL ALLOCATION PRIORITIES**

**Organic Growth Investments**

- High Return on Investment
- Impactful to long-term revenue CAGR

**External Innovation**

- Long-term growth focus
- High shareholder return
- Targeting deals <$1.5B with appropriate risk profile

**Return of Capital to Shareholders**

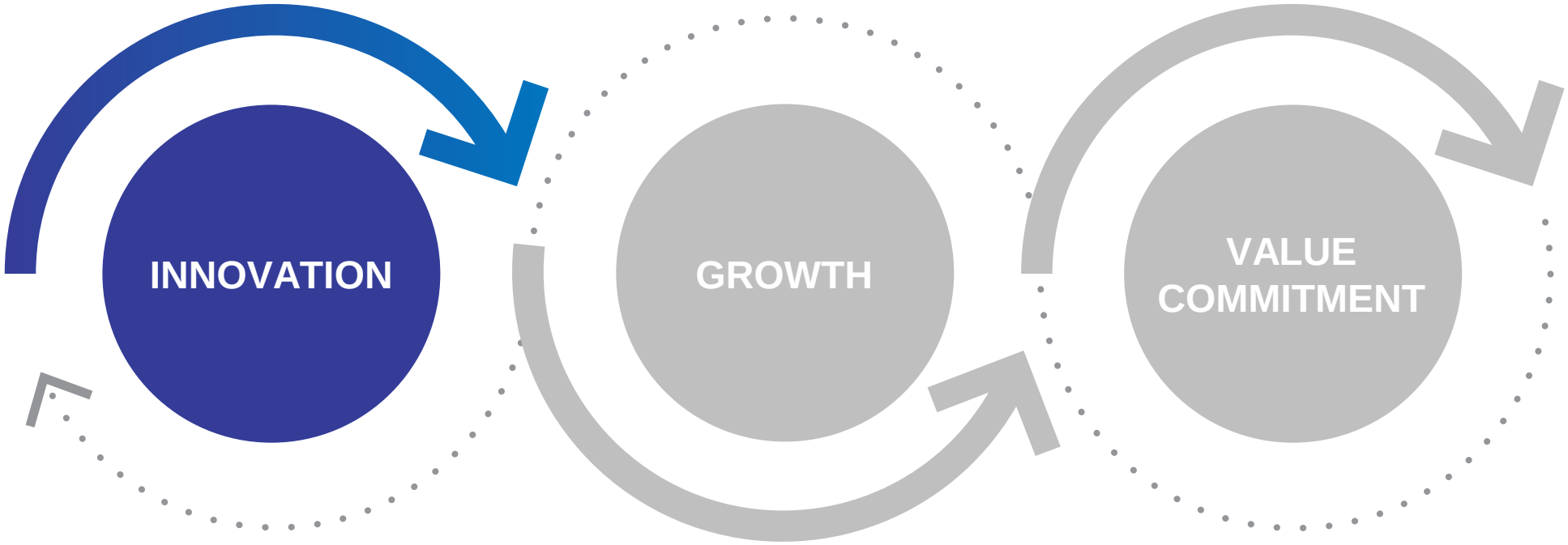
- Increasingly available tool
- Opportunistic liability and share count management

*$1.8B includes cash, cash equivalents, and investments; ~$500M convertible debt paid in August 2024, settled in cash

# Innovation Strategy that Supports Sustainable Pipeline of High Impact Medicines

**HENRY J. FUCHS, M.D.**
President, Worldwide Research & Development

# Our Strategy to Deliver Significant Value Creation




- Differentiated innovation engine
- Prioritized R&D pipeline
- Sustainability driven by genomics revolution

- Enzyme Therapies revitalized growth strategy
- VOXZOGO as sustainable growth driver in achondroplasia alone
- 5 new VOXZOGO indications advancing

- Accelerating profitability
- Increasing operating cash flow
- Business development to augment growth

# 25 Years of Translating Discoveries into Transformative Medicines

| | DISEASE | FIRST IN DISEASE | GENETIC | APPROVAL YEAR | MODALITY |
|---|---|---|---|---|---|
| ALDURAZYME (LARONIDASE) | MPS I | ✓ | ✓ | 2003 | Biologic |
| Naglazyme (GALSULFASE) | MPS IV | ✓ | ✓ | 2005 | Biologic |
| KUVAN (sapropterin dihydrochloride) Tablets or Powder for Oral Solution | PKU | ✓ | ✓ | 2007 | Small Molecule |
| VIMIZIM (elosulfase alfa) | MPS IVA | ✓ | ✓ | 2014 | Biologic |
| Brineura (cerliponase alfa) | CLN2 | ✓ | ✓ | 2017 | Biologic |
| Palynziq (pegvaliase-pqpz) Injection | PKU | 2nd (To Kuvan) | ✓ | 2018 | Biologic |
| VOXZOGO (vosoritide) for injection | Achondroplasia | ✓ | ✓ | 2021 | Peptide |
| ROCTAVIAN (valoctocogene roxaparvovec-rvox) | Hemophilia A | | ✓ | 2022 | Gene Therapy |

**~90% Post-Phase 2* Success Rate in the Last 20 Years**

*Post-Phase 2 studies conducted by BioMarin

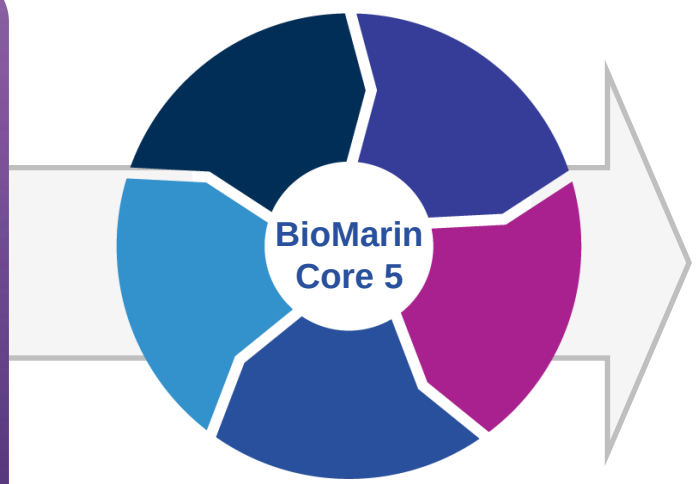
# Innovation Strategy is Sustainable, Replicable and Scalable

## BioMarin Innovation Engine

Universe of opportunity enabled by **Genomic Revolution**

Stringent filtration through **BioMarin's Core 5**

Leveraging our **distinct scientific, regulatory, and commercial capabilities** to create a sustainable pipeline of transformative medicines

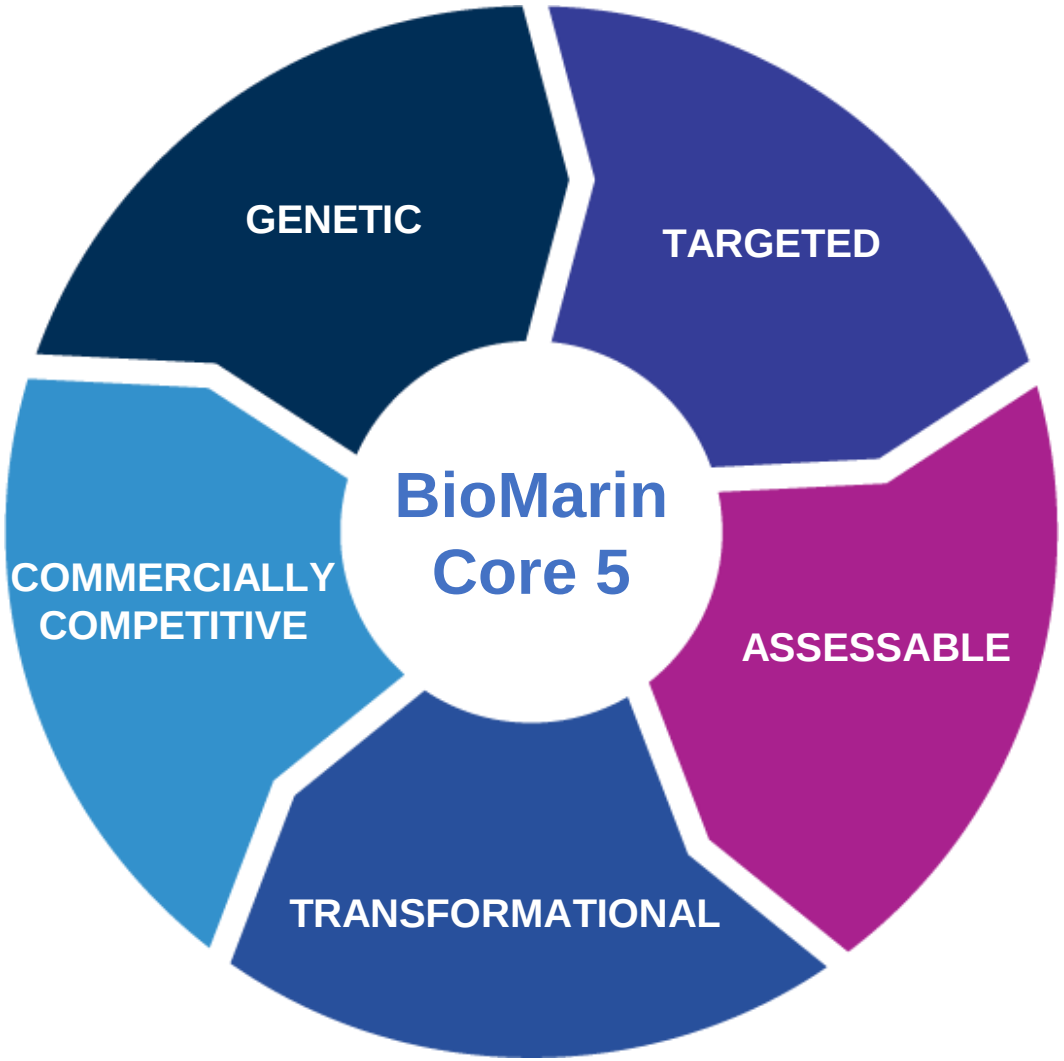



| | Candidate | Condition | Preclinical | Phase 1 | Phase 2 | Phase 3 | TAPP |
|---|---|---|---|---|---|---|---|
| Skeletal Conditions | VOXZOGO | Hypochondroplasia | | | | | ~14K |
| | VOXZOGO | Idiopathic Short Stature | | | | | ~190K |
| | VOXZOGO | Noonan Syndrome | | | | | ~90K |
| | VOXZOGO | Turner Syndrome | | | | | ~65K |
| | VOXZOGO | SHOX Deficiency | | | | | ~40K |
| | BMN 333 | ACH & HCH | | | | | ~38K* |
| Enzyme Therapies | PALYNZIQ | Phenylketonuria (ages 12-17) | | | | | ~7K |
| | BMN 390 | Phenylketonuria (Novel PEG) | | | | | ~150K |
| Innovation | BMN 351 | Duchenne Muscular Dystrophy | | | | | ~10K |
| | BMN 349 | Alpha-1 Antitrypsin Deficiency | | | | | ~95K |
| | BMN 370 | von Willebrand Disease | | | | | ~100K |

Additional 13 exploratory assets to support long-term growth

**More Opportunities** **Larger Populations**

# Core 5 Enables Transformational Impact




**GENETIC**
Clear etiology in affected populations

**TARGETED**
Therapeutic targets proximal to the fundamental defect

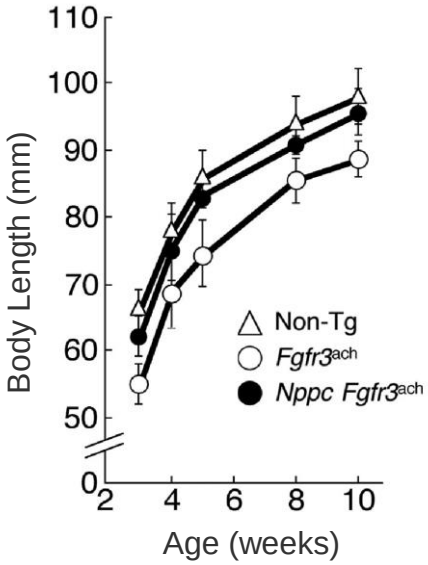
**ASSESSABLE**
Readily assessable endpoints that predict clinical benefit early

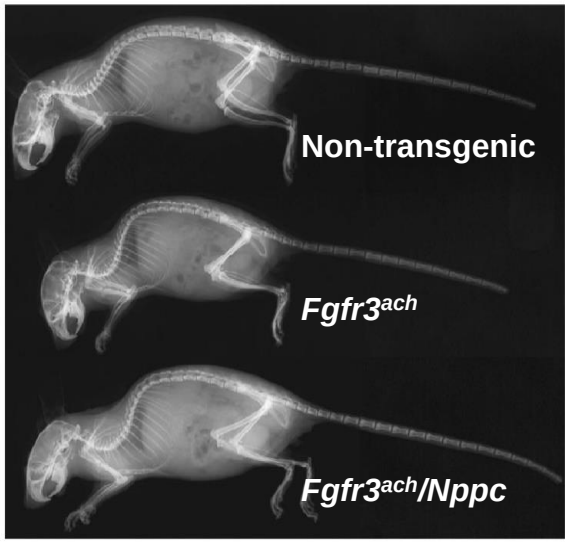
**TRANSFORMATIONAL**
Differentiated impact on patient lives

**COMMERCIALLY COMPETITIVE**
Compelling end-to-end commercial and competitive profile

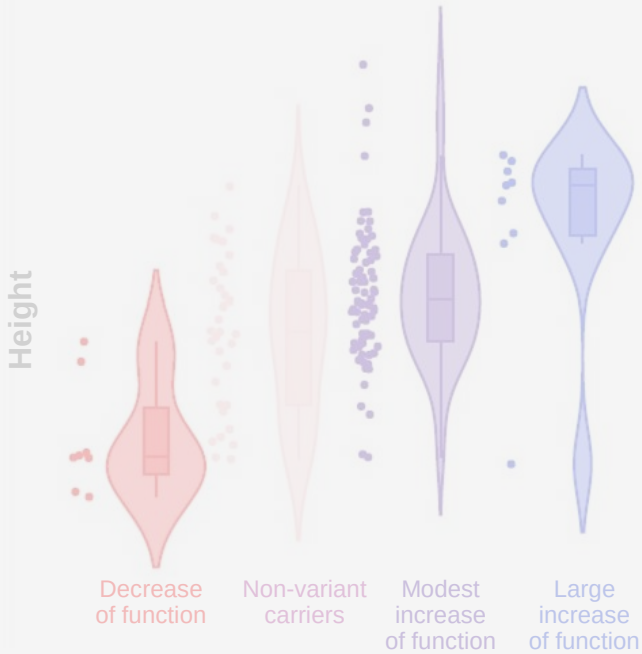
# C-natriuretic Peptide (CNP) Shows the Power of Genomic Discovery

## Overexpression of CNP "genetic cure" in achondroplasia models[1]




## Genetic evidence of CNP as master regulator of growth[2]




Color categories represent binned cGMP functional readout

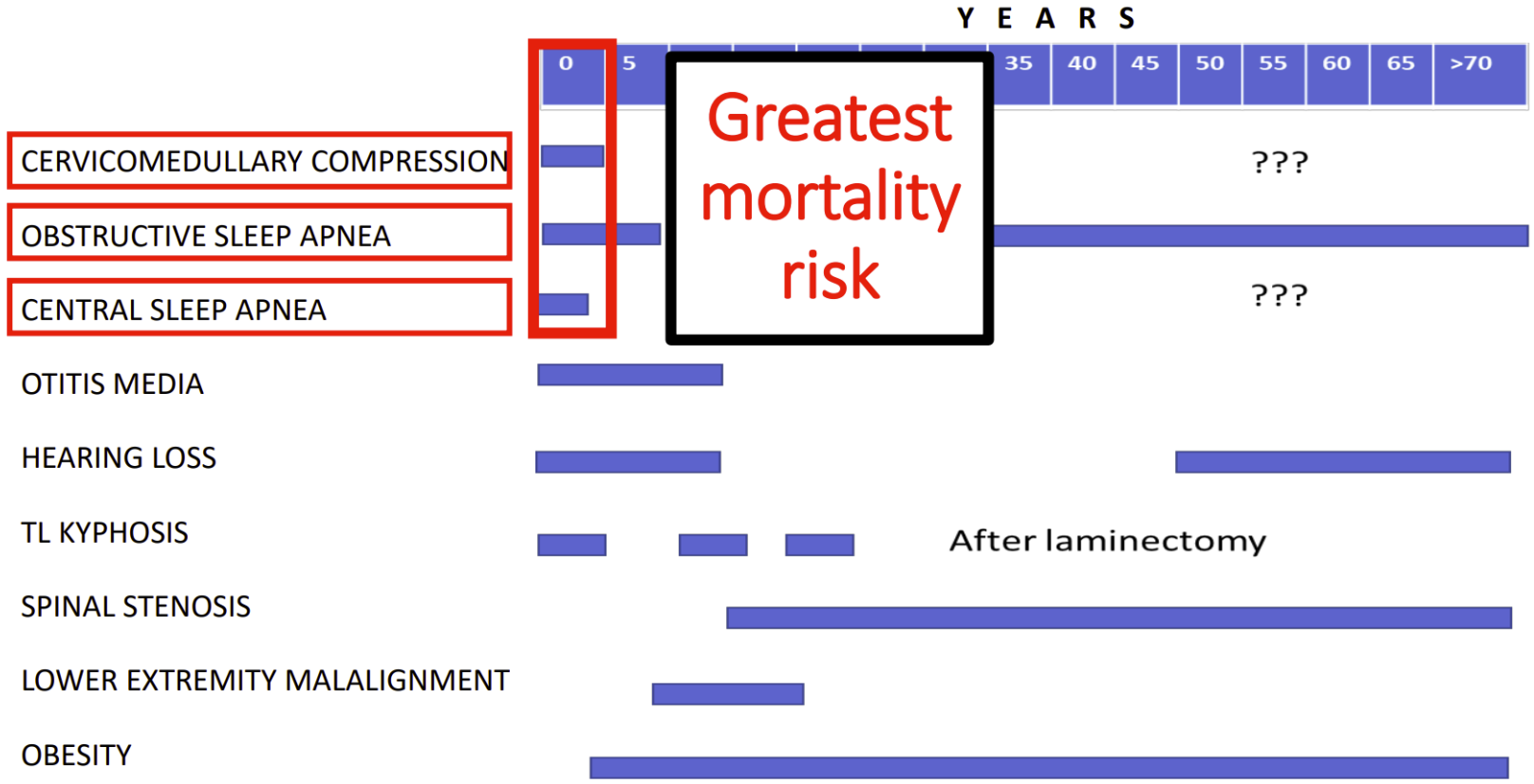
Boxes = median and inter-quantile range

Whiskers = 95% CI

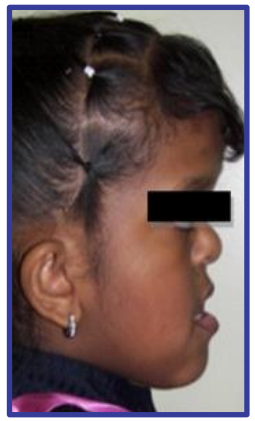
(1) See appendix slide 73, reference 1
(2) See appendix slide 73, reference 2

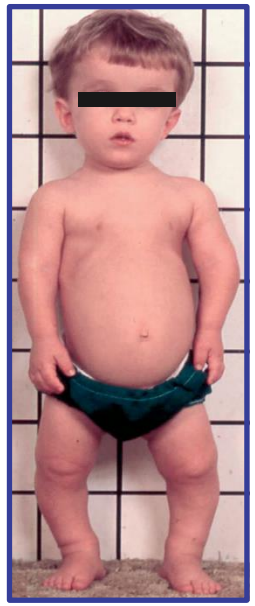
# Achondroplasia (ACH) – The Most Common Form of Dwarfism

## Life threatening risks, infancy to ~3 years of age




Hoover-Fong J.; *Bone* (2021). v146. https://doi.org/10.1016/j.bone.2021.115872

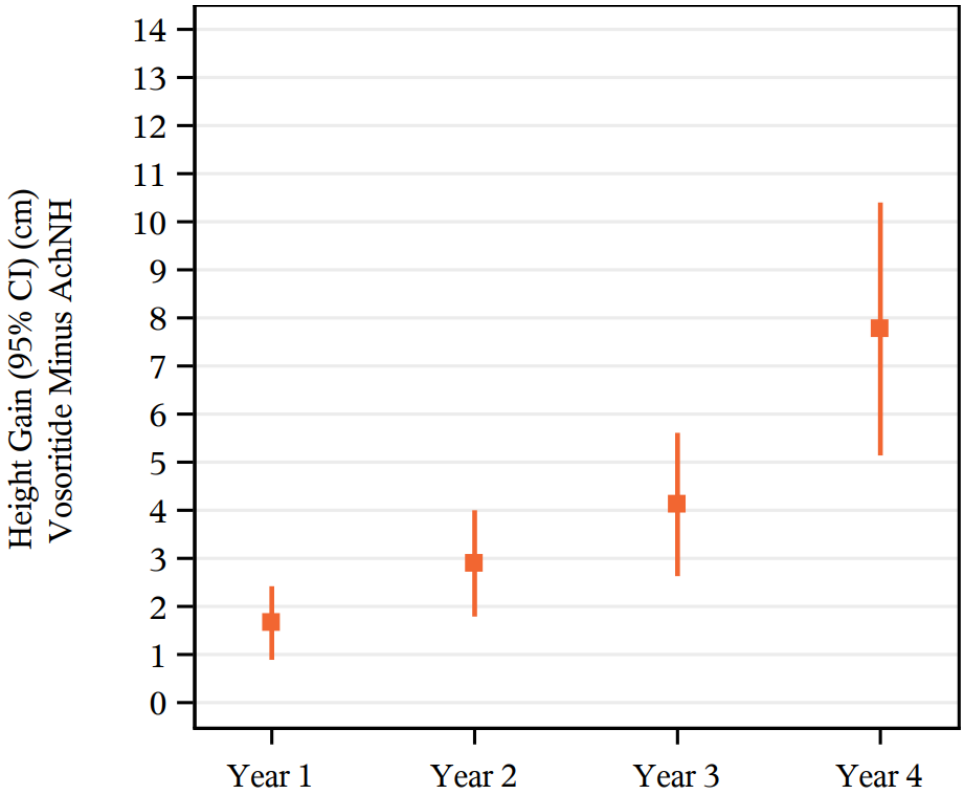




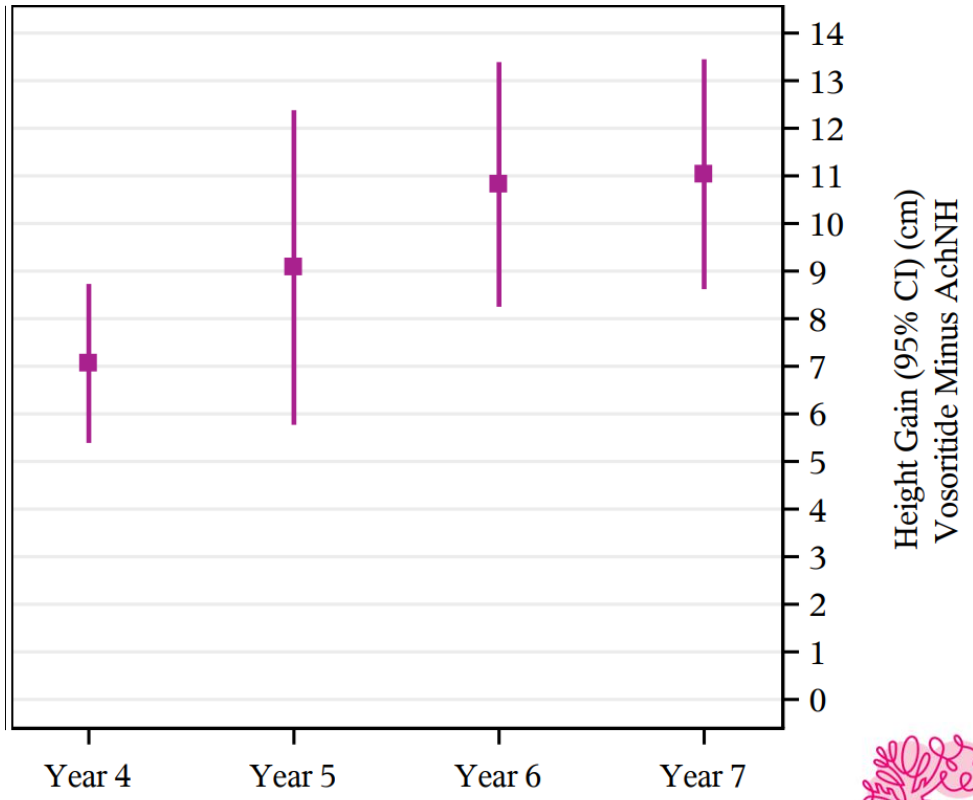
# VOXZOGO Ph 2: Expected Final Adult Height Gain >17cm (6.7in)

**Integration of Data from Two Separate Phase 2 Studies Suggest Cumulative Height Gain of at least 17cm**

## Phase 2: <5 years old study




## Phase 2: 5 years and older study

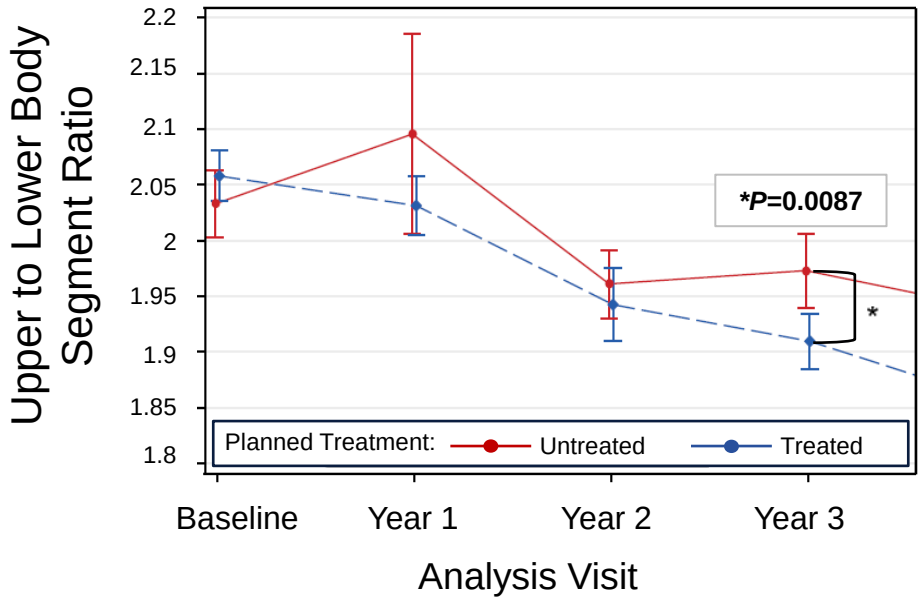



CANOPY ACH

# VOXZOGO: Multiple Benefits Beyond Height in Achondroplasia

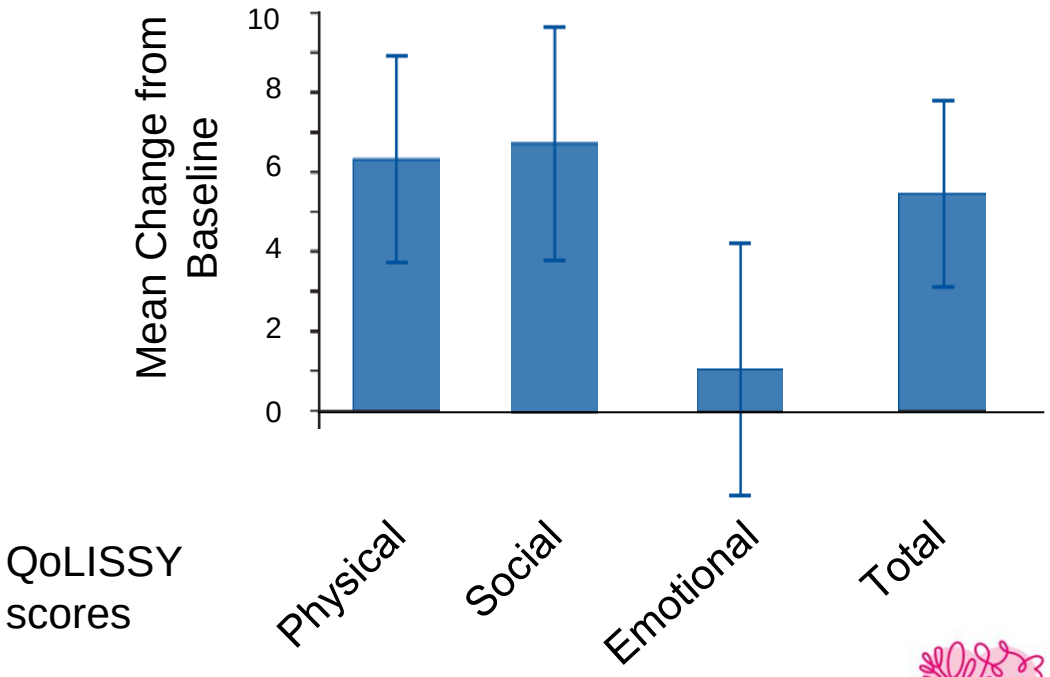
**Phase 3 Data**

## Proportionality Improved at Year 3 (n=48)[3]




**Phase 3 Data**

## Quality of Life Improved at Year 3 (n=119)[4]



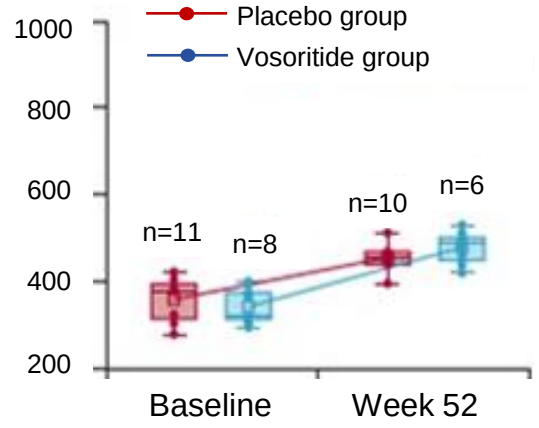

(3) See appendix slide 73, reference 3
(4) See appendix slide 73, reference 4

# VOXZOGO: Multiple Benefits Beyond Height in Achondroplasia

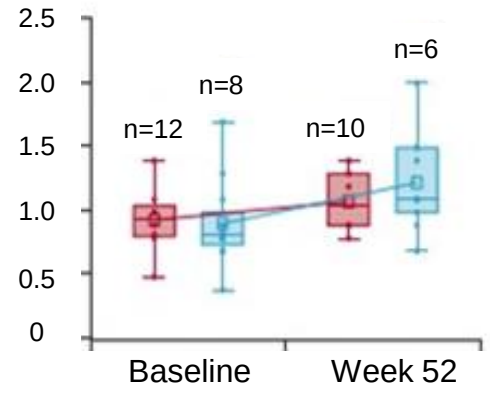
## Phase 2 Data

### Anatomical Improvements[5]

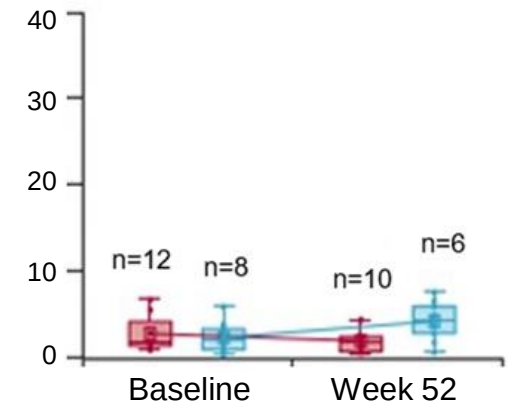
**Volume of face ($cm^3$)**




**Area of foramen magnum ($cm^2$)**



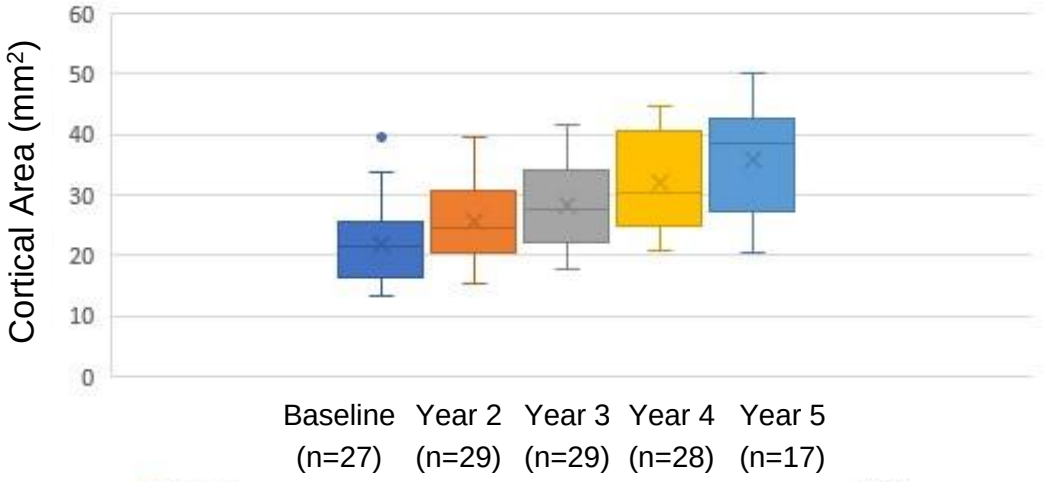

**Volume of sinus ($cm^3$)**




## Phase 3 Data

### Bone strength maintained during growth[6]

**Metacarpal Cortical Area ($mm^2$) By Timepoint**




(5) See appendix slide 73, reference 5
(6) See appendix slide 73, reference 6

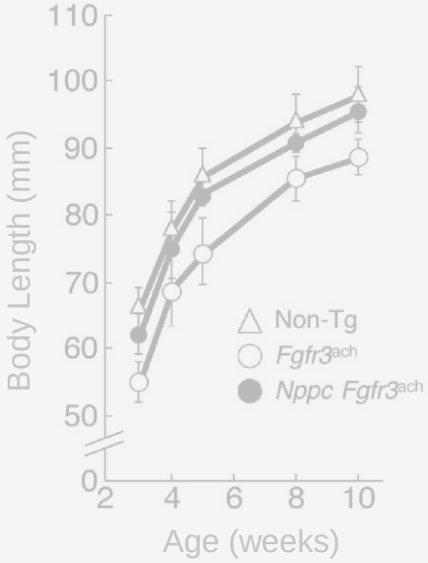
# Compelling Rationale for CNP

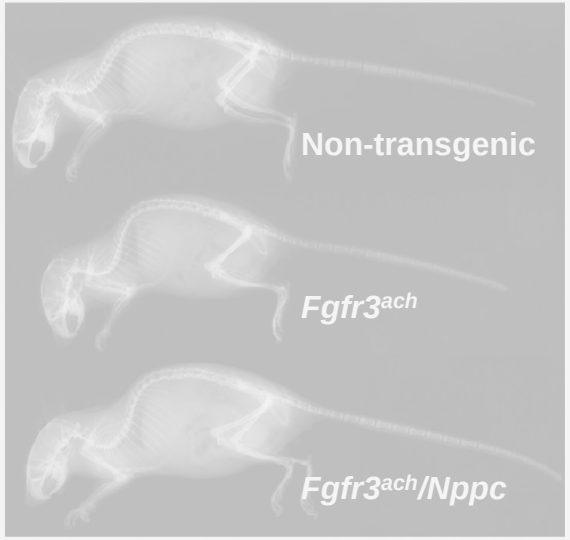
| | CNP (VOXZOGO)[7-16] | FGFR Inhibitor[17-18] |
|---|---|---|
| **On Market** | Globally since 2021 | ✗ |
| **Safety Experience** | ~6,000 patient years | ~18 patient years |
| **Demonstrated Durability** | 7 years ongoing | 18 months ongoing |
| **Health Beyond Height** | Proportionality, Quality of Life, Bone Structure, and Function | Limited Proportionality |
| **Eligibility under 3 years** | Globally since 2023 | Uninvestigated |
| **Alterations in Phosphate Balance** | None | Observed |
| **Route of Administration** | SubQ | Oral |
| **Multiple Indication Potential (MOA)** | Broadly applicable | Limited |

(7-16) See appendix slide 73, references 7-16
(17-18) See appendix slide 73, reference 17-18

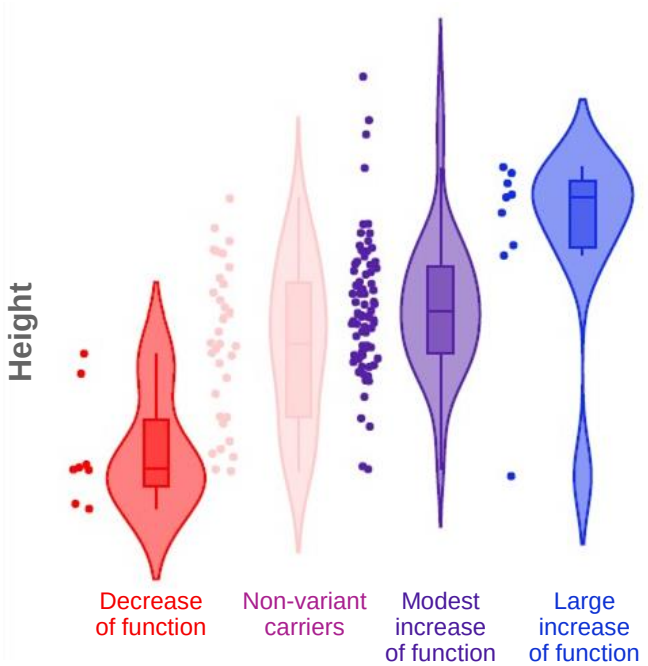
# CNP for Multiple Indications Embodies the Core 5

## Overexpression of CNP "genetic cure" in achondroplasia models[1]




## Genetic evidence of CNP as master regulator of growth[2]




Color categories represent binned cGMP functional readout

Boxes = median and inter-quantile range

Whiskers = 95% CI

(1) See appendix slide 73, reference 1
(2) See appendix slide 73, reference 2

# VOXZOGO in ACH Supported Rapid Development in Hypochondroplasia

## Hypochondroplasia

- Diagnosis often delayed
- Features can overlap both achondroplasia and idiopathic short stature
- No approved therapy (excl. Japan)
- Similar pathophysiology and burden of illness as achondroplasia
- Targeting most severely affected patient subset: height ≤ -2.0 SDs

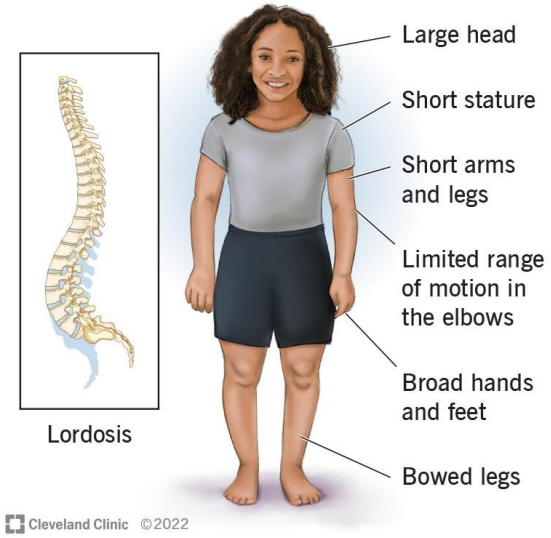
### Pivotal study underway

CANOPY HCH

**2026** Target Ph3 Data Readout

**2024** Ph3 FPI

**2027** Target Launch

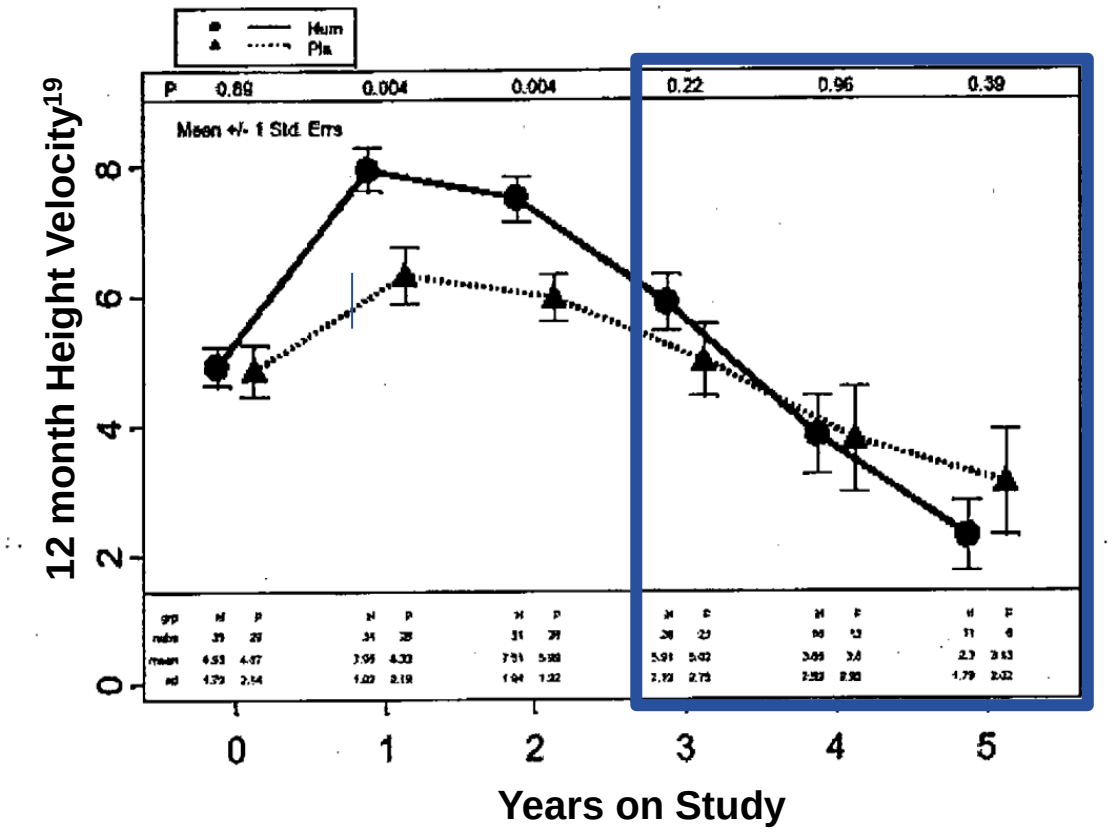



## Development Highlights in Hypochondroplasia

- **Data in hypochondroplasia at ACMG 2024 demonstrated a mean increase in AGV of 1.8 cm/year (n=24)**
- **Global health authorities aligned to direct-to-Phase 3 program supported by strong clinical evidence in achondroplasia**
- **Placebo-controlled annualized growth velocity (AGV) 52-week endpoint for potential registration**

# Limitations of Growth Hormone in non-Growth Hormone Deficient Conditions

## Waning Effect of Growth Hormone over Time




## Final adult height: Untreated vs. hGH treated

| Condition | Untreated Final Adult Height | Height Gain with hGH Treatment |
|---|---|---|
| Idiopathic Short Stature[20-21] | Female: <150 cm (4 ft 11 in)<br>Male: <160 cm (5 ft 3 in) | <7.5 cm (3 in) |
| Noonan Syndrome[22] | Female: <154.4 cm (5 ft 1 in)<br>Male: <169.2 cm (5 ft 7 in) | Female: ~9.2 cm (3.6 in)<br>Male: ~10.9 cm (4.3 in) |
| Turner Syndrome[23] | Female: <146 cm (4 ft 9 in) | ~6 cm (2.4 in) |
| SHOX Deficiency[24-25] | 135 cm (4 ft 5 in) to normal height | <10 cm (3.9 in) |

(19-25) See appendix slide 74, references 19-25

# VOXZOGO in Achondroplasia Facilitates Additional Expansions

**Gained regulatory alignment for 4 additional VOXZOGO indications**

**Placebo-controlled AGV 52-week endpoint for potential registration**

## Idiopathic Short Stature (ISS)

- hGH approved; efficacy wanes
- Mean increase in AGV of 5.2 cm/year (n=5) (PES 2024)
- Targeting most severely affected patient subset: height ≤ -2.5 SDs

### Phase 2 clinical study enrolling


## Noonan Syndrome, Turner Syndrome, SHOX Deficiency

- hGH approved; efficacy wanes
- Mean increase in AGV of 4.3 cm/year in Noonan Syndrome (n=3) (PES 2024)
- Targeting most severely affected patient subset: height ≤ -2.5 SDs

### Phase 2 clinical trial enrolling Q4'24

# BMN 333: Longer-acting CNP to Build on VOXZOGO

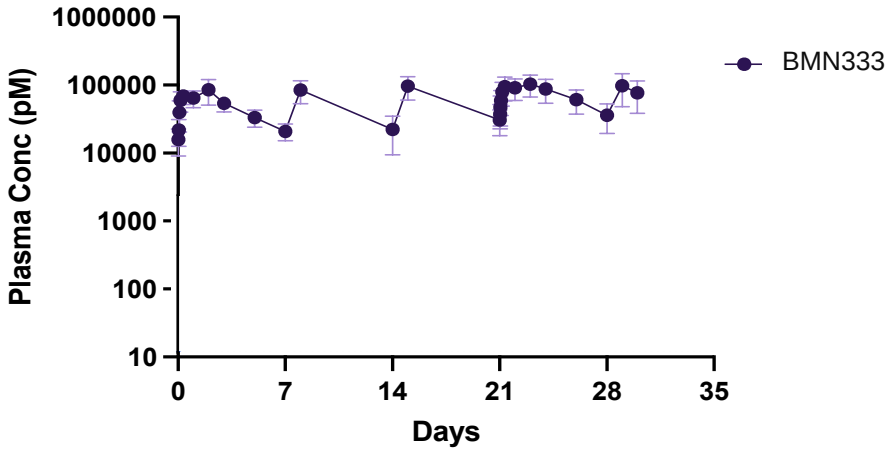
## BMN 333 – Longer-Acting CNP

- Leverages similar chemistry from other approved drugs for prolonging half-life and potential weekly dosing
- Offers the potential for **even better efficacy, convenience, or both from sustained exposure**
- Leverages extensive **safety experience** of VOXZOGO (same CNP)
- Extends **CNP value for patients**
- Patent portfolio covering multiple long-acting CNP variants in the U.S. and Europe*

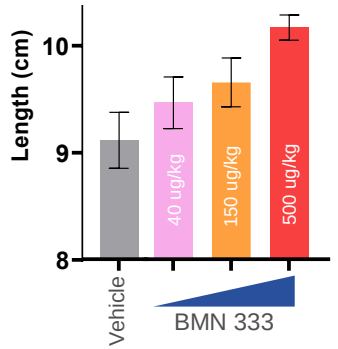
**Target IND submission: 4Q24** → **Target HV SAD: 2025** → **Target POC: 2026** → Target Launch **2030**

**TAPP: ~38K****

## Demonstrated safety in MOA for half-life extension




## BMN 333 intended to enable higher CNP exposure to potentially yield greater growth




*Daily dosing equivalent

*CNP38 patent confirmed following opposition proceedings at European Patent Office (opposition completed June 2024, EP3175863B1, exp May 2030). CNP38 patent confirmed following re-examination by U.S. Patent Office (re exam allowed June 2030; USRE48267, exp May 2030). CNP39 potential expiration in 2045 (US 8,198,242, EP2432489B1) based on possible patent term extension.
**Total Addressable Patient Population initially targeting achondroplasia and hypochondroplasia (duplicative with VOXZOGO TAPP)

# Focused Pipeline May Address Significant Patient Populations

| | Candidate | Condition | Preclinical | Phase 1 | Phase 2 | Phase 3 | TAPP |
|---|---|---|---|---|---|---|---|
| **Skeletal Conditions** | VOXZOGO | Hypochondroplasia | | | | | ~14K |
| | VOXZOGO | Idiopathic Short Stature | | | | | ~190K |
| | VOXZOGO | Noonan Syndrome | | | | | ~90K |
| | VOXZOGO | Turner Syndrome | | | | | ~65K |
| | VOXZOGO | SHOX Deficiency | | | | | ~40K |
| | BMN 333 | ACH & HCH | | | | | ~38K* |
| **Enzyme Therapies** | PALYNZIQ | Phenylketonuria (ages 12-17) | | | | | ~7K |
| | BMN 390 | Phenylketonuria (Novel PEG) | | | | | ~150K |
| **Innovation** | BMN 351 | Duchenne Muscular Dystrophy | | | | | ~10K |
| | BMN 349 | Alpha-1 Antitrypsin Deficiency | | | | | ~95K |
| | BMN 370 | von Willebrand Disease | | | | | ~100K |

Additional 13 exploratory assets to support long-term growth

Targeting enrollment of Ph2 patients in 2024

*BMN 333 initially targets achondroplasia and hypochondroplasia (duplicative with VOXZOGO TAPP)

# PALYNZIQ for Adolescents with Phenylketonuria (PKU) Addresses High Unmet Need

## PALYNZIQ – Adolescents with PKU (PAHD)

- PKU requires strict adherence to intolerable protein restriction
  - Untreated PKU:  cognitive and psychiatric impairment
  - Earlier therapy reduces dietary restriction and facilitates transition to adult living
- Genetic condition benefits from **early initiation**; requires adherence to strict diet
- Safety and benefit in **high-need population** (12-17y/o US, 12-15y/o ex-US) expected to be consistent with adult data
- Expands existing PALYNZIQ label
- Health authority-**endorsed study underway**
- Expansion unlocks market access, **strengthens value proposition**, compels payer support

**Target US sBLA filing: 2H25** → **Target EU Filing: 1H26** → Target Launch **2026**

**TAPP: ~7K***

**PATRICK COLEMAN**, adult living with PKU and now receiving PALYNZIQ[26]

(9g protein roughly equivalent to 1.5 eggs or 0.5cup Greek yogurt)

"I was allowed **nine grams of protein a day when I was 16 years old** and playing four sports."

*As an adult on PALYNZIQ therapy, Patrick's sentiment is…*

"I was like, you know what, **I want to try a lamb chop and it was probably the best thing I have ever put in my mouth."**

*TAPP eligibility: Ages 12-17; PHE > 600
(26) See appendix slide 74, reference 26

# BMN 390: May Strengthen Profile vs. PALYNZIQ and Expand Uptake

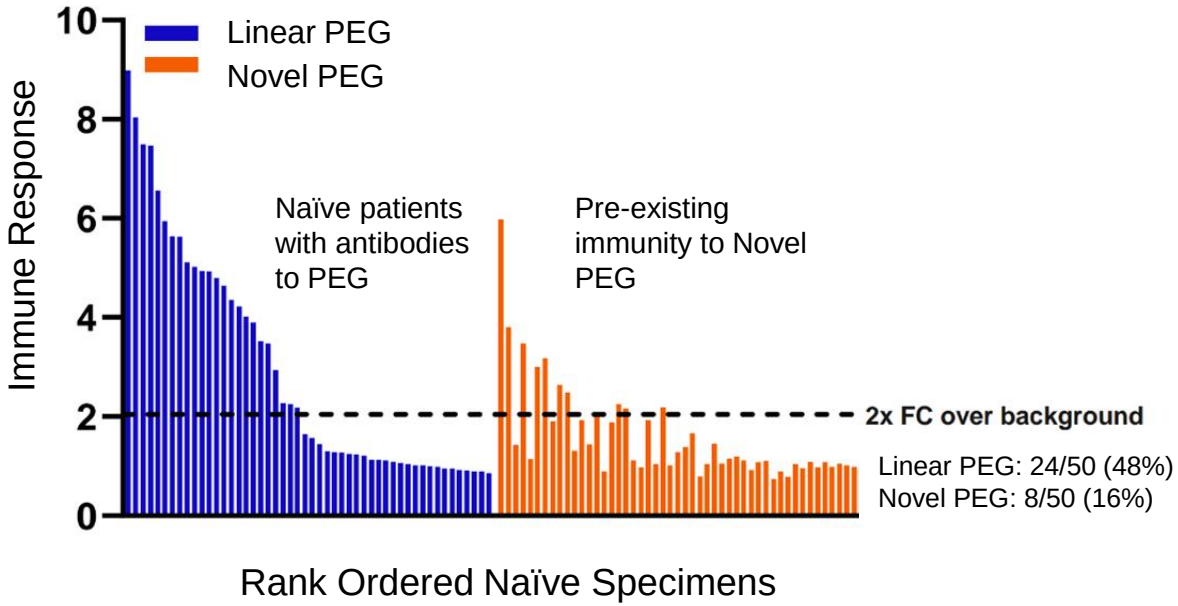
## BMN 390 – Phenylketonuria (PKU/PAHD)

- Potential to **greatly expand** the number of patients who can benefit from enzyme therapy
  - Through lower hypersensitivity
  - Expansion into younger ages
- Reduced immunogenicity from novel pegylation
- Leverages PHE as an **approvable endpoint**
- **Potential to replace diet as mainstay of early therapy of PKU/PAHD**

**Target IND 2H25** → **Target POC 2029** → **Target Ph3 Start: 2030** → Target Launch **2034**

**TAPP: ~150K***

## Novel PEG is less immunoreactive than linear PEG in polymer-naïve human donor sera



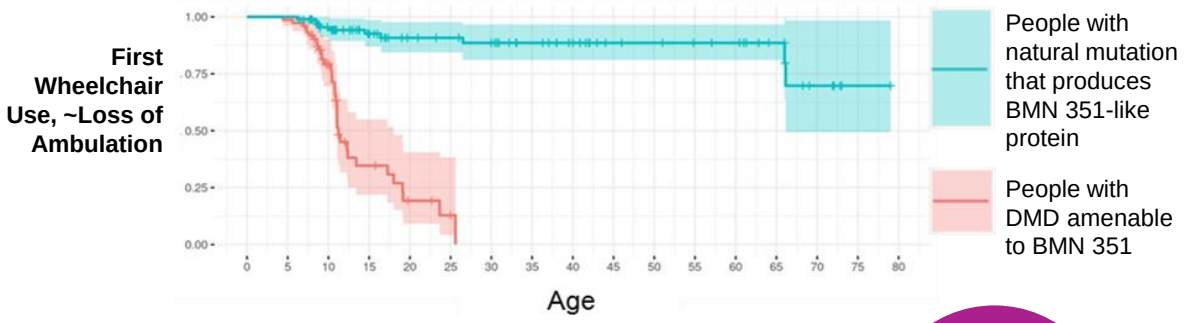

PAHD = Phenylalanine hydroxylase deficiency; PAHD is now being adopted in place of PKU to refer to the same condition
*TAPP eligibility: ages 5+

# BMN 351: Novel Target to Enable More Dystrophin Expression

## BMN 351 – Duchenne Muscular Dystrophy

- Genetic insights revealed unique **novel site** for exon skipping
- Novel compound leverages first-generation clinical experience
- Potent near full-length dystrophin expression up to 40%, multiples higher than approved therapy

**Anticipated transformative benefit of BMN 351**

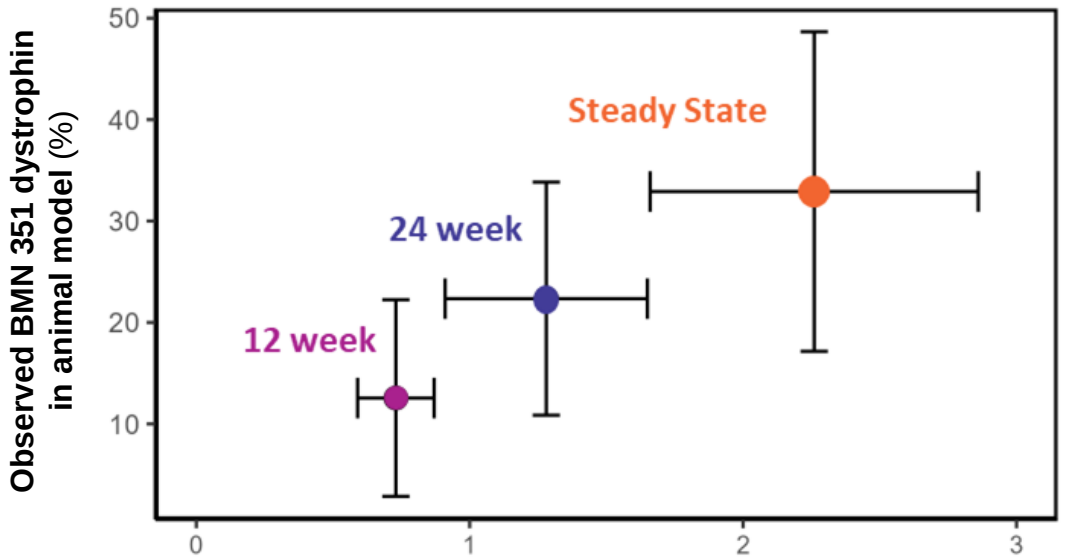



**Phase 1/2 2024** → **Target POC 2025** → **Target Ph3 Start: 2026** → Target Launch **2028**

**TAPP: ~10K***

*TAPP eligibility: Males with DMD amenable to Exon 51 skipping

## Similar chemistry, better biology



# BMN 349: Potential Best-in-disease treatment for A1AT Liver Disease

## BMN 349 – Alpha-1 Antitrypsin Deficiency

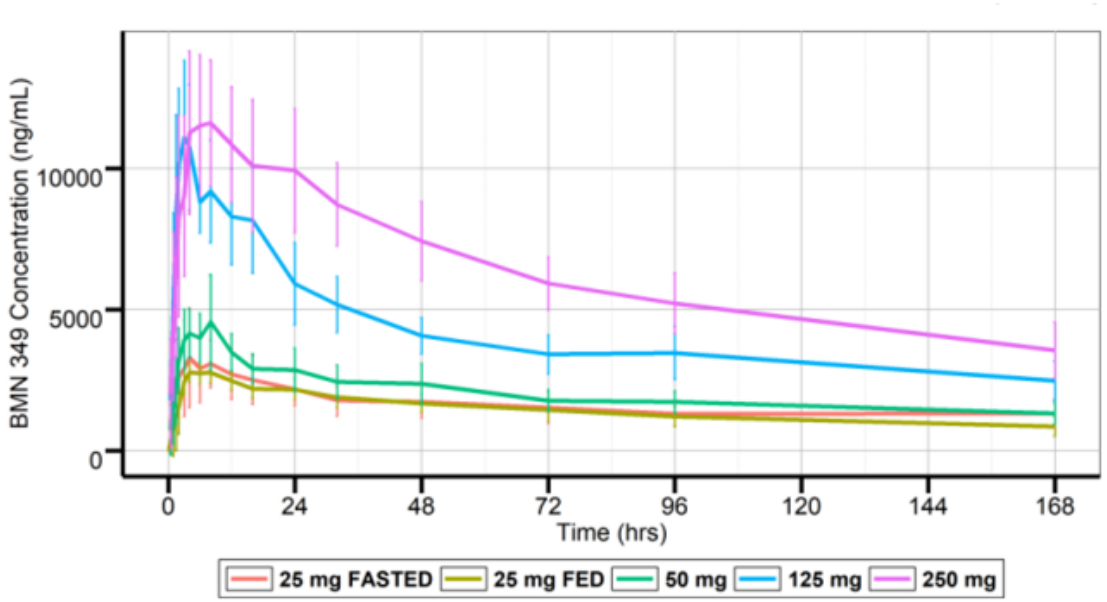
- Dysfunctional A1AT protein causes liver and lung disease
- **BMN 349 targets aggregates that cause liver disease and complements approved replacement therapy for lung disease**
- **Unique MOA** "stabilizes" mutant protein to address fundamental pathology
- Increase protein secretion reflecting reduced polymer burden
- Potential to transform liver health
- **Potential competitive advantages**
  - **Uniquely applicable to broader heterozygous population**
  - **Orally titratable to readily achieve correct level of effect**

**Phase 1 2024** → **Target POC 2026** → **Target Ph3 Start: 2028** → Target Launch **2033**

**TAPP: ~95K***

*TAPP eligibility: ages 18+; ZZ with F2+ Liver Disease and MZ with F4 Liver Disease

## Total BMN 349 exposures in healthy volunteers by dose



# BMN 370: Opportunity to Transform Treatment of von Willebrand Disease

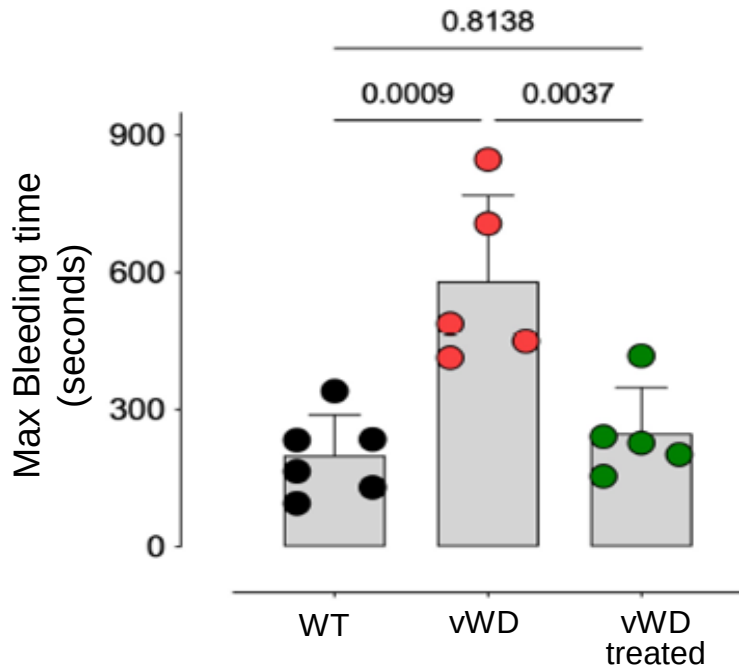
## BMN 370 – von Willebrand Disease (vWD)

- vWD causes severe bleeding during menstruation and childbirth; comorbidities
- **BMN 370 has potential to restore physiologic expression of clotting protein without perturbing other elements of hemostasis**
- **Targeted** bi-specific nanobody for subQ administration replaces 3x per week IV therapy
- Plasma vWF levels **readily assessable**
- **Transformational** potential to normalize vWF levels with a monthly at-home self-administered injection

**Target IND 2H25** → **Target POC 2029** → **Target Ph3 Start: 2030** → Target Launch **2034**

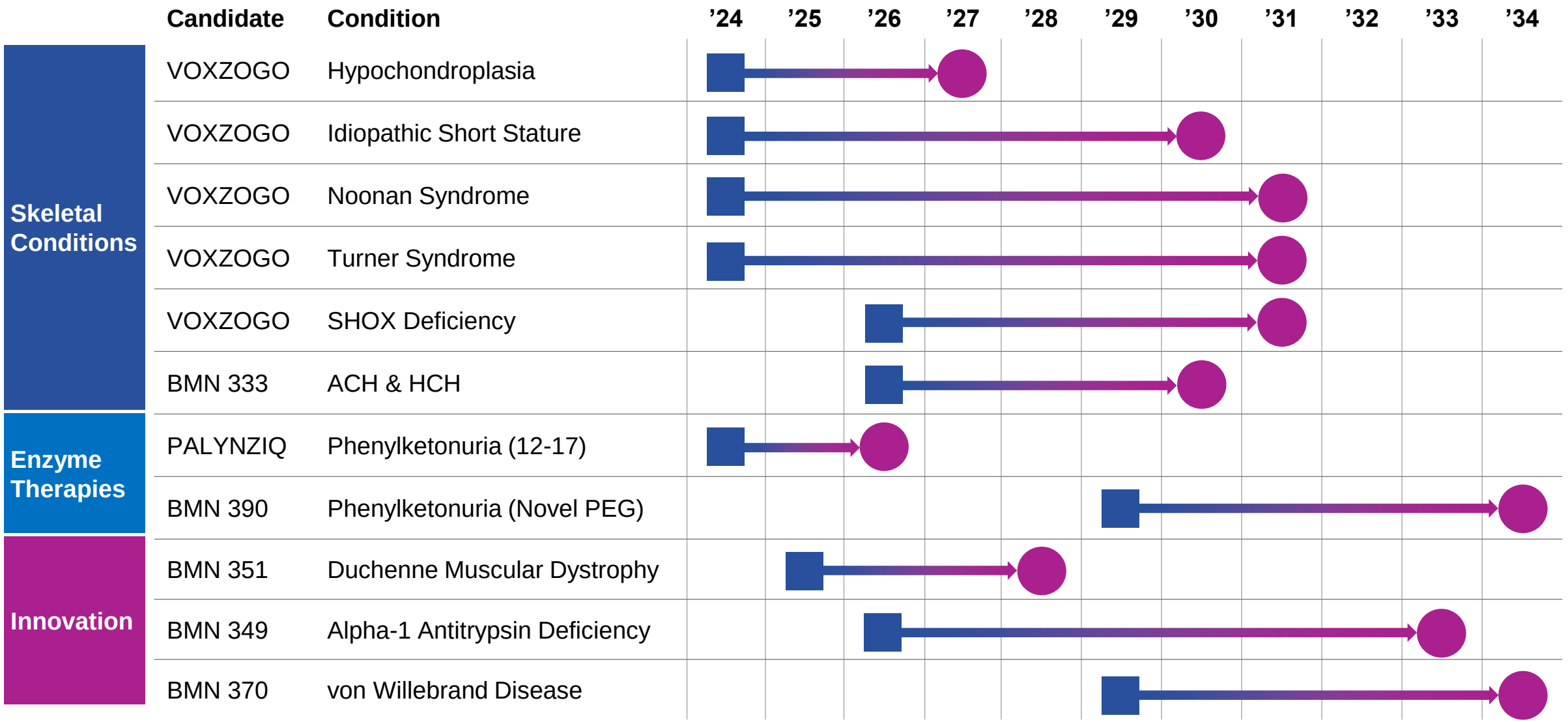
**TAPP: ~100K***

## Single SQ injection normalizes bleeding phenotype in a vWD mouse model




*TAPP eligibility: Females with Type 1 vWD and Moderate-to-Severe Menorrhagia

# Targeting 11 Launches by 2034, Two Launches by 2027



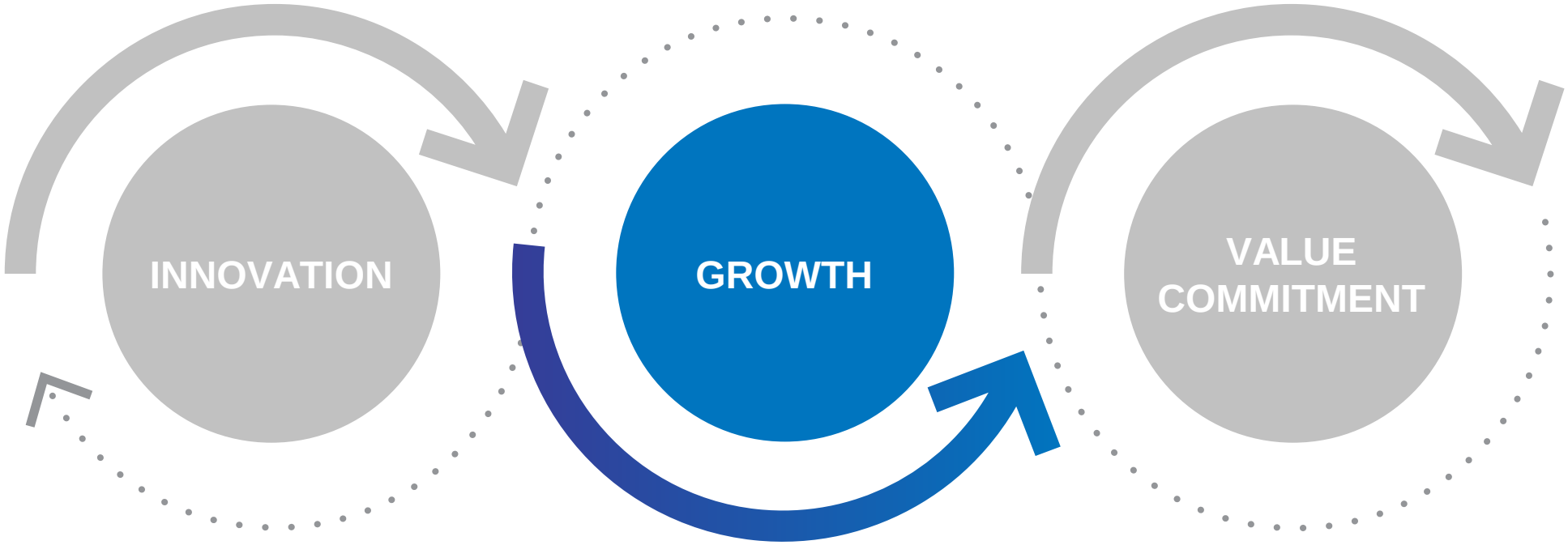
# Growth:

## Optimizing BioMarin's Growing and Durable Enzyme Therapies Business Unit

## Building Leadership in Achondroplasia To Set the Stage for Multiple New Indications

**CRISTIN HUBBARD**
Executive Vice President and
Chief Commercial Officer

# Our Strategy to Deliver Significant Value Creation

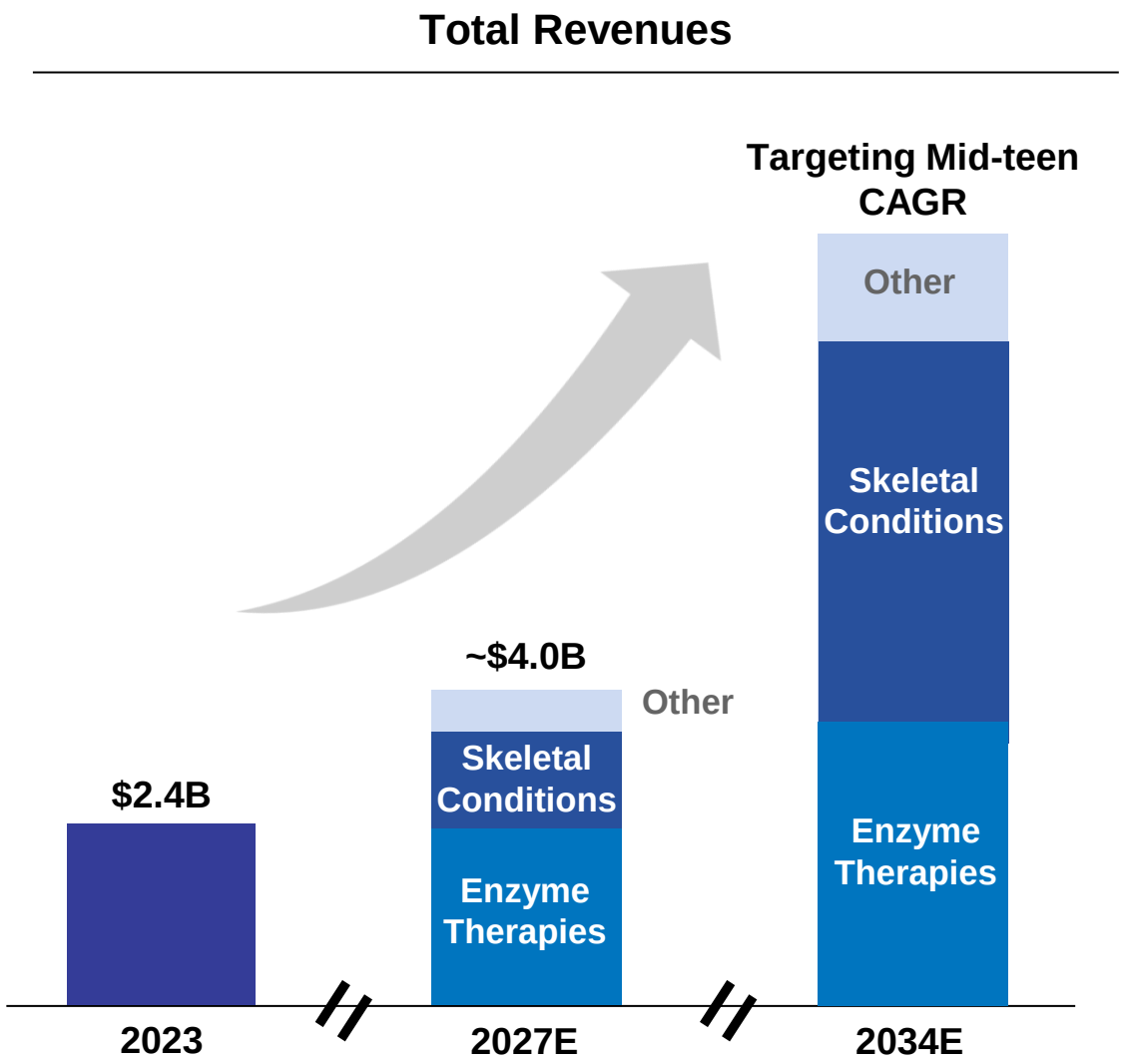



- Differentiated innovation engine
- Prioritized R&D pipeline
- Sustainability driven by genomics revolution

- Enzyme Therapies revitalized growth strategy
- VOXZOGO as sustainable growth driver in achondroplasia alone
- 5 new VOXZOGO indications advancing

- Accelerating profitability
- Increasing operating cash flow
- Business development to augment growth

# Approved Brands Driving Most Long-term Revenue Growth




## Enzyme Therapies

- Strong Underlying Foundation of Growth
- Sustained Market Leadership with Current Portfolio
- Insulated from Inflation Reduction Act

## Skeletal Conditions

- VOXZOGO ACH Market Leadership and Continued Expansion
- Five new indications with VOXZOGO:
  - HCH Global Launch Expected in 2027
  - ISS, Noonan Syndrome, Turner Syndrome, and SHOX Deficiency Expected in 2030 and Beyond
- BMN 333 Long-acting CNP Expected in 2030

# Enzyme Therapies: Market Leader with Global Capabilities

## Established Differentiated Capabilities

Identifying Patients and Creating Access Pathways

## Built Global Commercial Infrastructure

Supports Patients in Healthcare Systems Worldwide

## Established & Sustained Leadership

Standard of Care Across All Indications

VIMIZIM™ (elosulfase alfa)

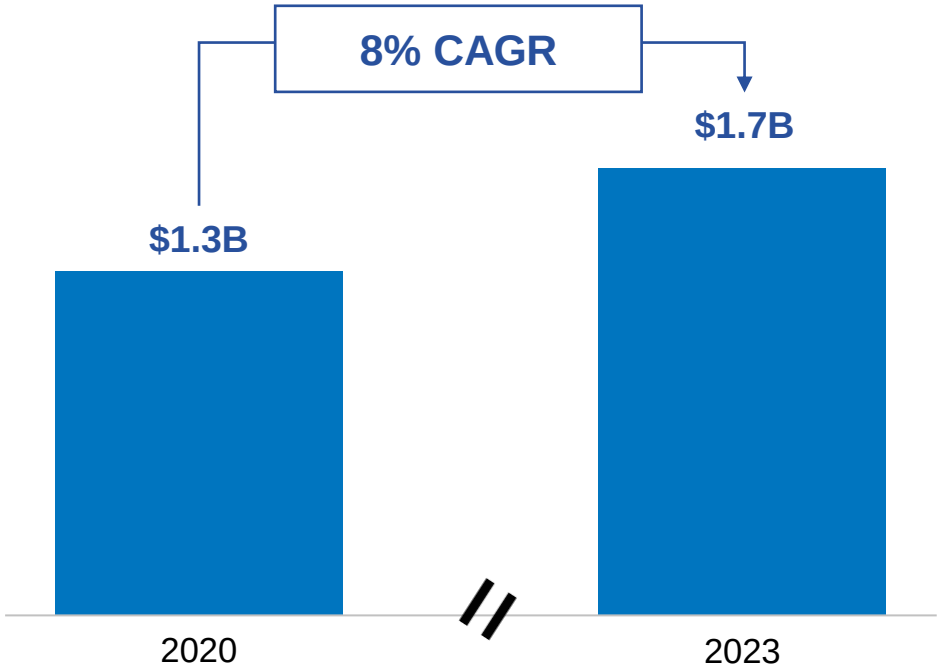
Naglazyme® (GALSULFASE)

Palynziq® (pegvaliase-pqpz) Injection

Brineura® (cerliponase alfa)

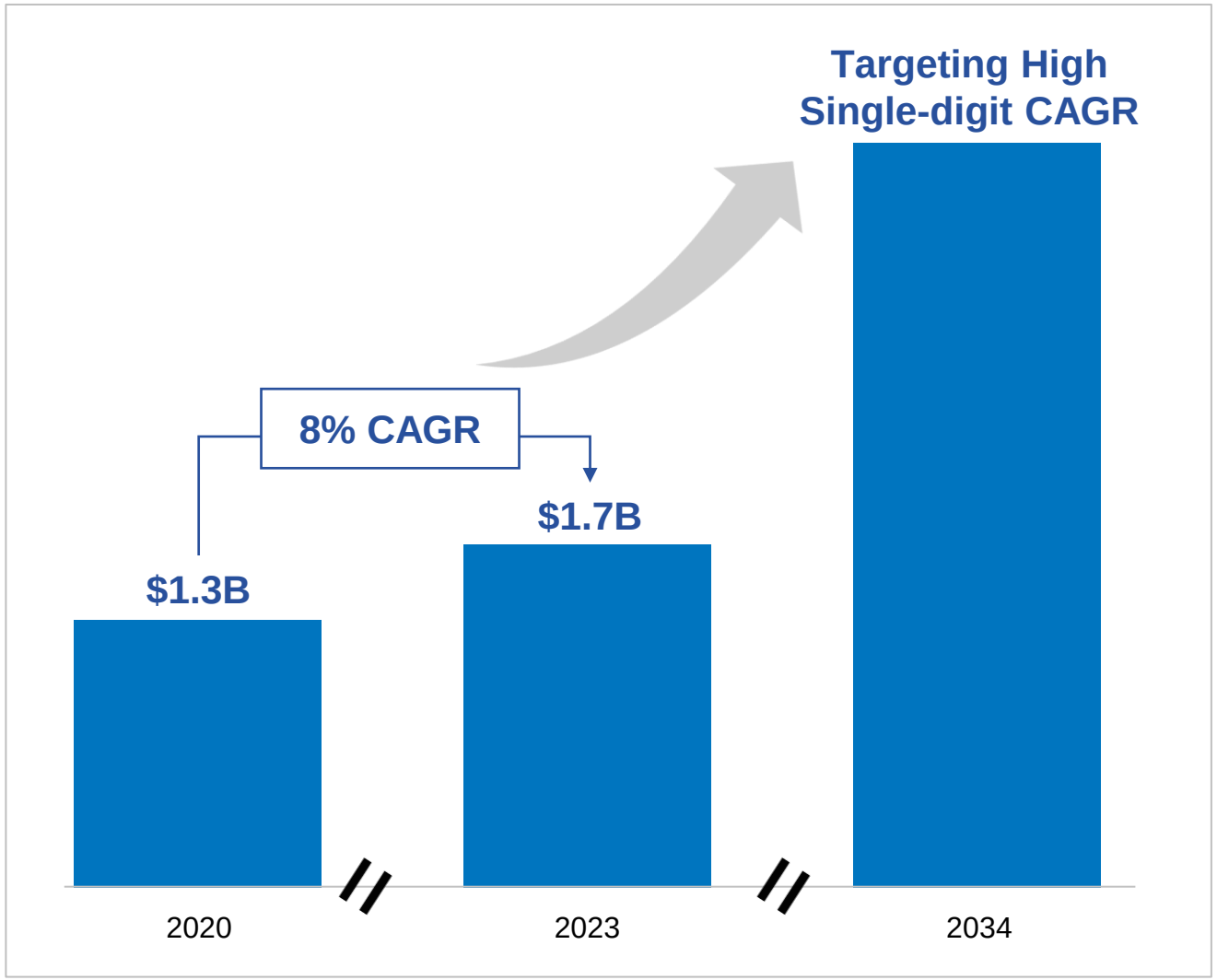
ALDURAZYME® (LARONIDASE)

### Enzyme Therapies Revenue



# Enzyme Therapies Leader with Expansion Opportunities

## Enzyme Therapies Revenue




VIMIZIM™ (elosulfase alfa)

Naglazyme® (GALSULFASE)

Palynziq® (pegvaliase-pqpz) Injection

Brineura® (cerliponase alfa)

ALDURAZYME® (LARONIDASE)

# Enzyme Therapies: New Initiatives to Drive Growth

## Increase Diagnosis

Resourcing targeted diagnostic efforts to **find more patients**

**Broader testing across family tree** once patient has been diagnosed

## Enhance Patient Adherence

**Maintain patient adherence above 90%** with patient support and home infusion programs

Increase compliance with improved **convenience measures**

## Invest in Emerging Markets

Unlock growth opportunities in **emerging markets**

**Tailor investments** to support diagnosis and access in these regions

## Palynziq Differentiation

**Adolescent indication** launch expected in 2026, expanding eligible patients by **10%**

**Ongoing evidence generation** to create opportunities for **further geographic expansion**

VIMIZIM™ (elosulfase alfa)

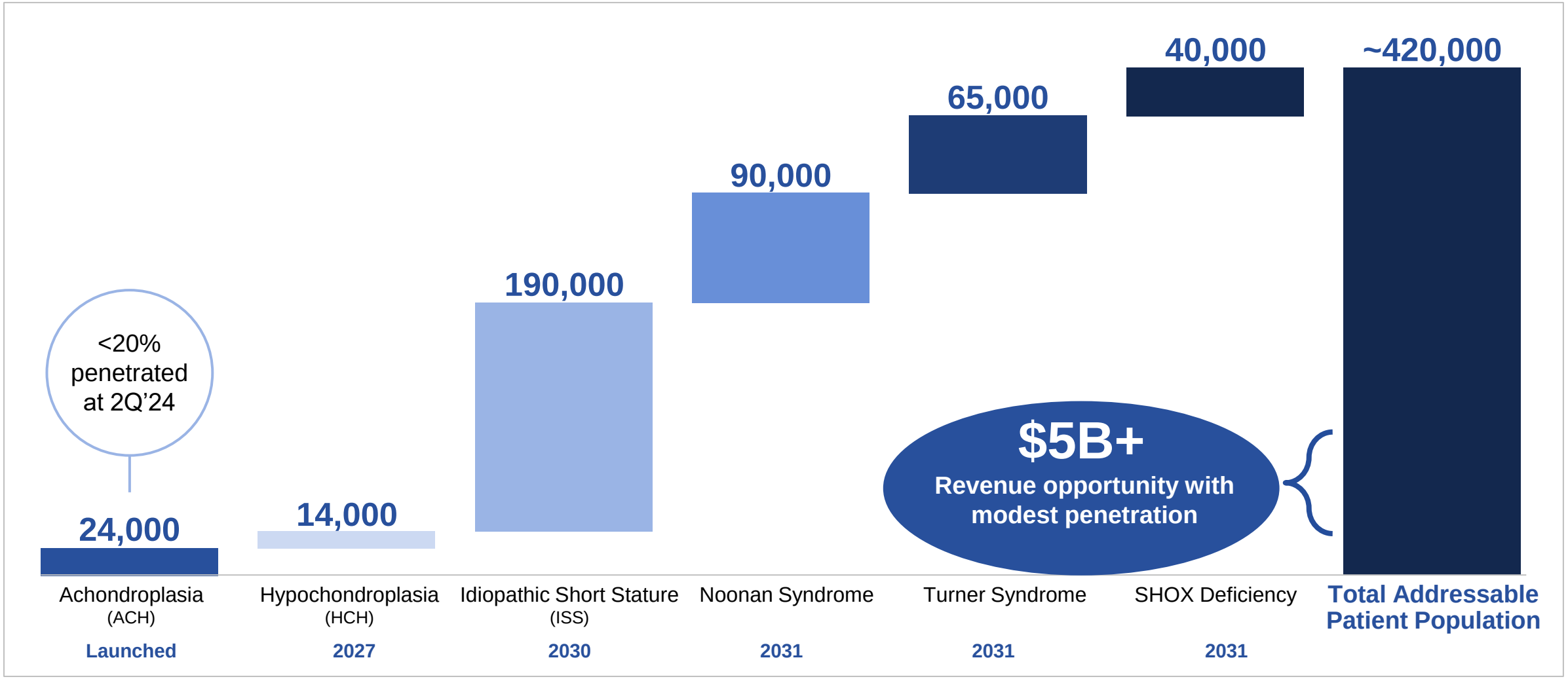
Naglazyme® (GALSULFASE)

Brineura® (cerliponase alfa)

Palynziq® (pegvaliase-pqpz) Injection

# Multiple New Indications Leverage VOXZOGO® Leadership

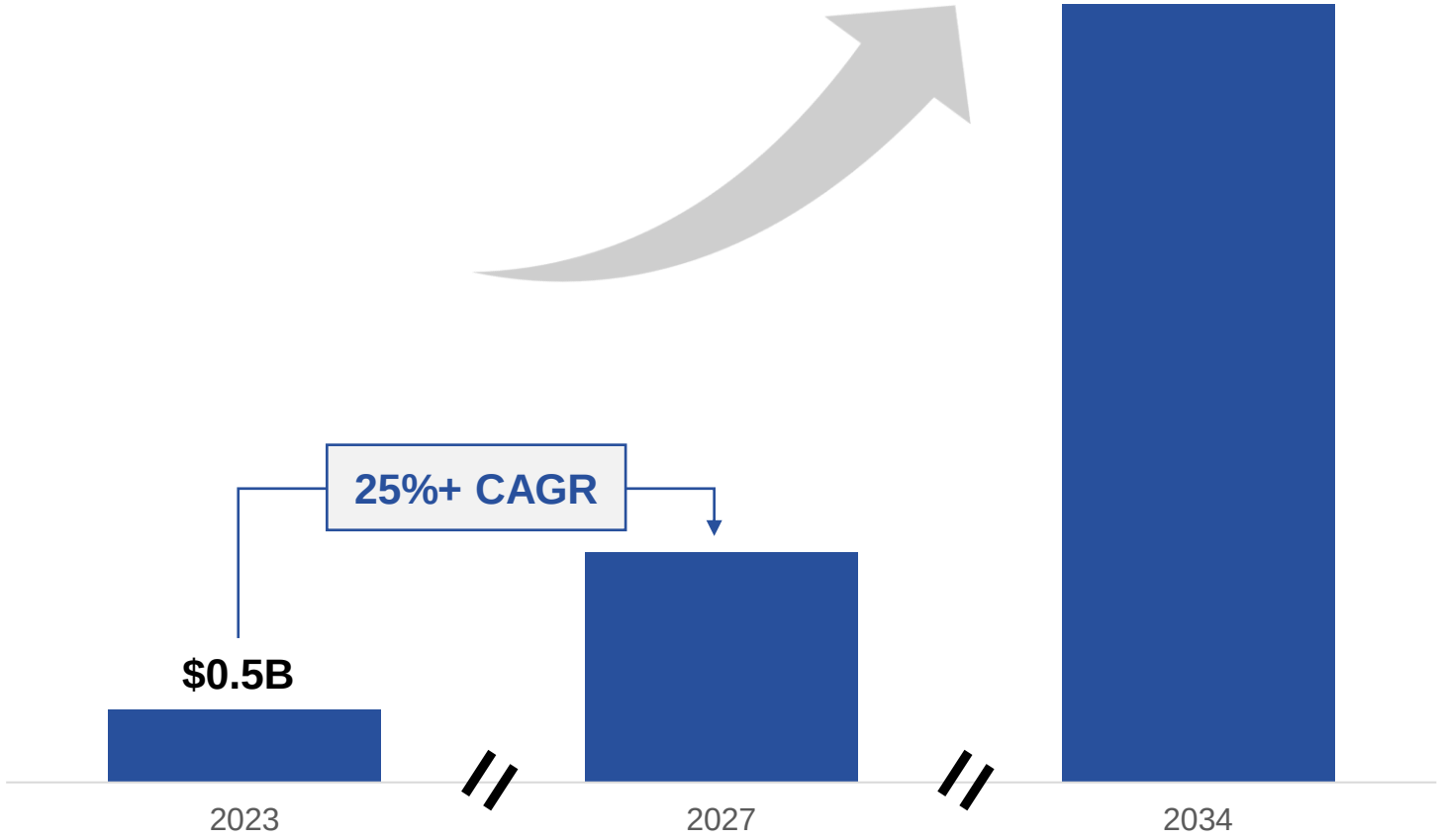
## Total Addressable Patient Population (TAPP)*




*TAPP defined as the diagnosed, clinically eligible patients for a given product or program in a defined footprint; footprint for above indications (except ISS) defined as all markets included in BioMarin's internal projections for VOXZOGO revenues. ISS footprint is US-only. Height Z-score eligibility for TAPP: ACH (N/A); HCH (≤ -2.0 standard deviations (SDs)); ISS (≤ -2.5 SDs); Noonan Syndrome (≤ -2.5 SDs); Turner Syndrome (≤ -2.5 SDs); SHOX Deficiency (≤ -2.5 SDs)

# Confident Outlook in Skeletal Conditions

## Skeletal Conditions Revenue



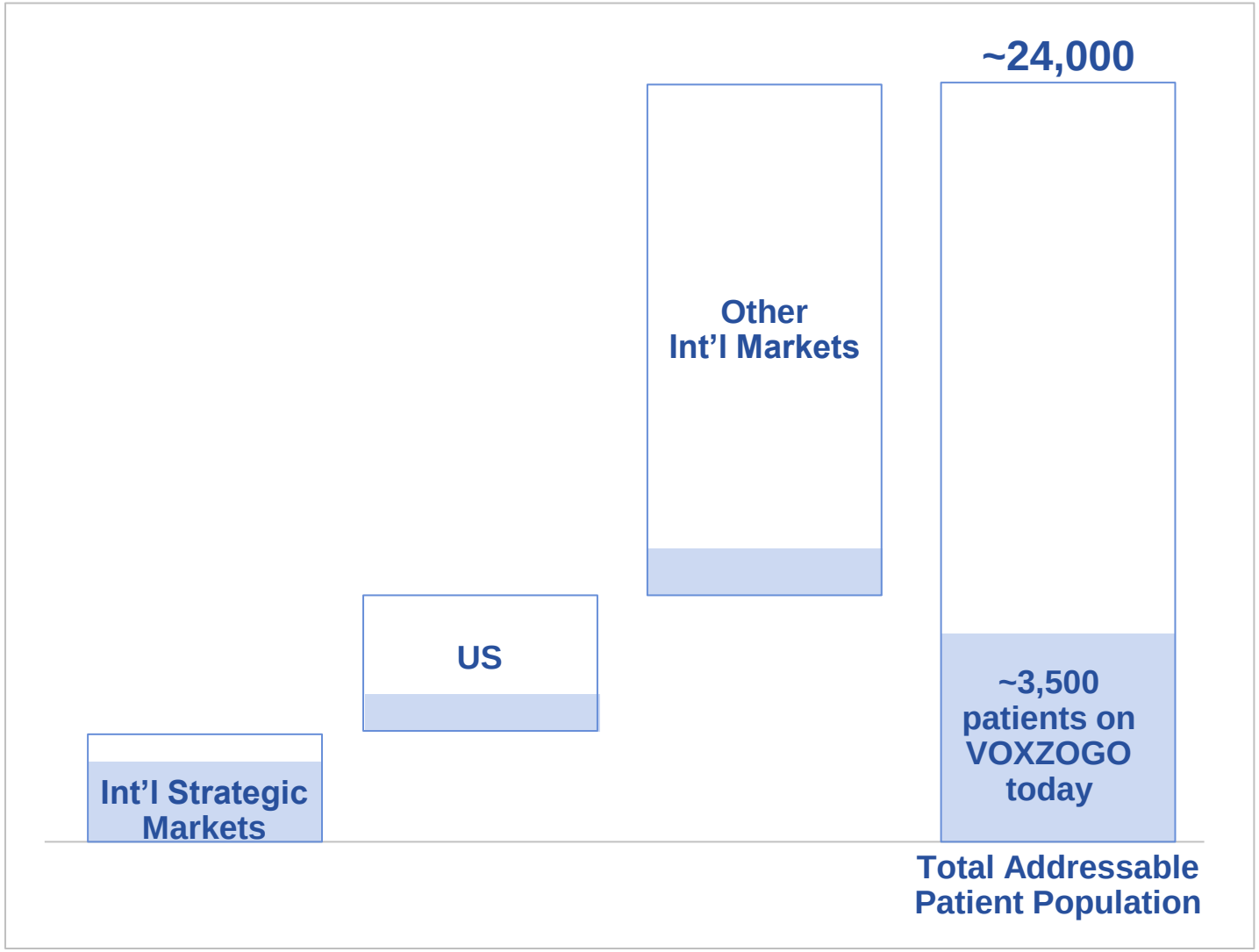

## VOXZOGO: Transforming the Treatment of Achondroplasia

- 6,000 patient years of robust efficacy and safety data
- Only approved precision therapy for infants and children across the globe
- Current experience demonstrates high compliance rates (>95%)
- Market research suggests reluctance to switch from VOXZOGO if treatment is demonstrating strong effect
- Patent portfolio includes multiple variants of long-acting CNP across key geographies
  - Recent European ruling on CNP38 patent* increases confidence around our intellectual property

*CNP38 patent confirmed following opposition proceedings at European Patent Office (opposition completed June 2024, EP3175863B1, exp May 2030). CNP38 patent confirmed following re-examination by U.S. Patent Office (re exam allowed June 2030; USRE48267, exp May 2030). CNP39 potential expiration in 2045 (US 8,198,242, EP2432489B1) based on possible patent term extension.

# Expanding Our Leadership with VOXZOGO® in Achondroplasia

**VOXZOGO is expected to be the only precision treatment option until at least 2027**



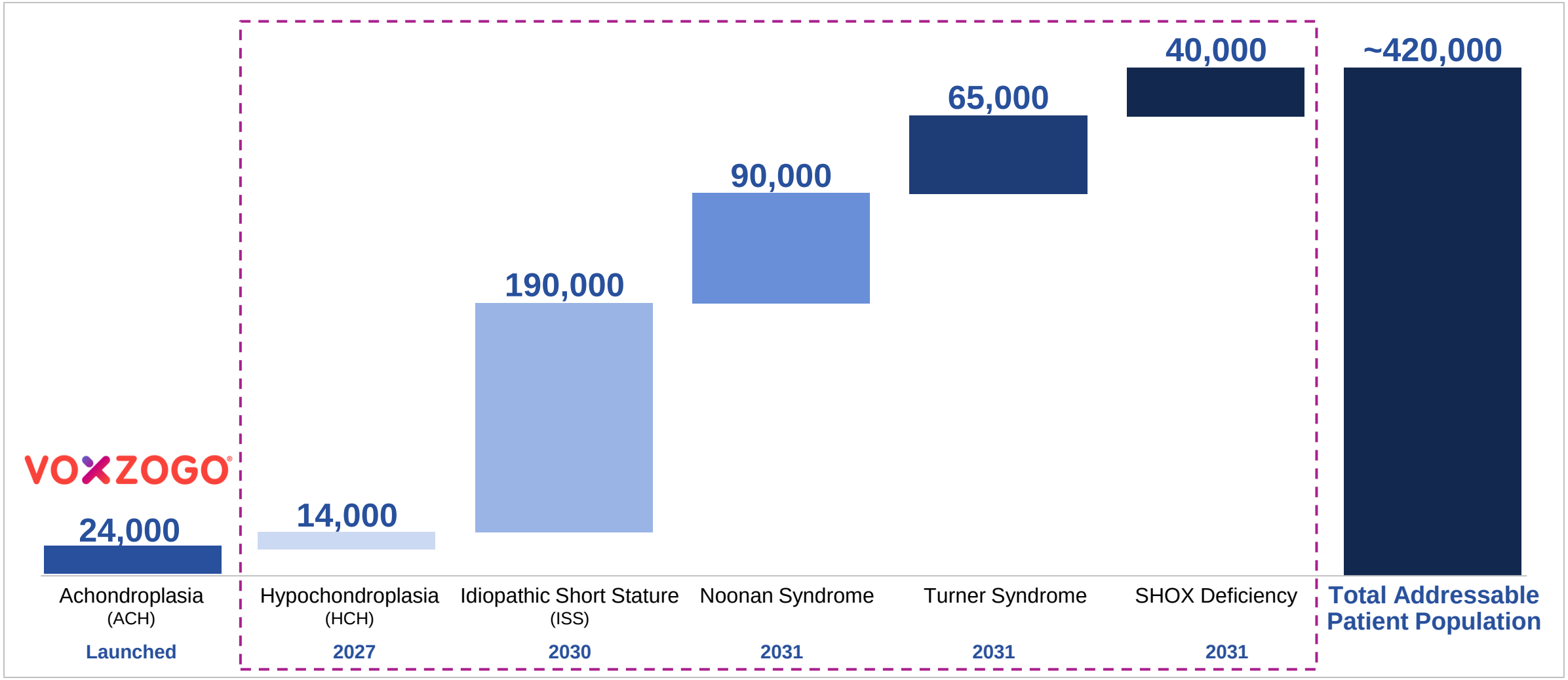

## Strategies to Expand Reach

- **Continued expansion** in strategic markets (e.g. Japan, Germany and Italy)
  - Leveraging high awareness and penetration rates to further increase eligible patient uptake
- Building on our **increasing prescriber base** and **recently expanded age label** to optimize U.S. market penetration
  - In largest single market, use data-driven insights and digital channels to increase awareness and build on momentum from age label expansion
- **Patient additions** in under-penetrated markets and **opening new markets**
  - Commercialized in 44 markets with plans to expand into >20 new markets by 2027

~3,500 VOXZOGO patients as of end of 2Q'24

# Multiple New Indications Leverage VOXZOGO® Leadership

## Total Addressable Patient Population (TAPP)*




*TAPP defined as the diagnosed, clinically eligible patients for a given product or program in a defined footprint; footprint for above indications (except ISS) defined as all markets included in BioMarin's internal projections for VOXZOGO revenues. ISS footprint is US-only. Height Z-score eligibility for TAPP: ACH (N/A); HCH (≤ -2.0 standard deviations (SDs)); ISS (≤ -2.5 SDs); Noonan Syndrome (≤ -2.5 SDs); Turner Syndrome (≤ -2.5 SDs); SHOX Deficiency (≤ -2.5 SDs)

# VOXZOGO® Potential 1st Precision Medicine for Hypochondroplasia

**Target Launch: 2027**

## Open Opportunity in New Indication

High unmet need population and under-diagnosed

Features can overlap both achondroplasia and idiopathic short stature

No currently approved targeted therapies for HCH (excl. Japan)

VOXZOGO® (vosoritide) for injection

**Hypochondroplasia (HCH)**

## Market Strategy

Leverage experience in achondroplasia: market creation, KOL relationships, prescriber base

Increase diagnosis and establish treatment paradigm

## Population Background

Total addressable patient population (TAPP): ~14,000

TAPP eligibility includes those with height Z-score ≤ -2.0 SDs

Prevalence: ~4.6 per 100,000

# VOXZOGO Potential to Outperform Current Standard of Care in Idiopathic Short Stature

**Target Launch: 2030**

## Opportunity to Outperform hGH

VOXZOGO mechanism of action has proof of concept in idiopathic short stature

Current hGH treatment effect wanes in 70-80% of patients[27]

Demonstrated durability of VOXZOGO has the potential to drive better outcomes

No expected competition from FGFR inhibitors

VOXZOGO (vosoritide) for injection

**Idiopathic Short Stature**

## Market Strategy

Target established growth hormone market

Leverage our increasing leadership in ACH and HCH

## Population Background

Total U.S. addressable patient population: ~190,000

TAPP eligibility includes those with height Z-score ≤ -2.5 SDs

Prevalence: ~410 per 100,000

(27) See appendix slide 74, reference 27

# VOXZOGO® May Have Transformative Potential in Three Additional Indications with High Unmet Need

## Target Launches: 2031

| | VOXZOGO® Noonan Syndrome ~90,000* | VOXZOGO® Turner Syndrome ~65,000* | VOXZOGO® SHOX Deficiency ~40,000* |
|---|---|---|---|
| **Prevalence:** | **~40 per 100,000** | **~32 per 100,000** | **~9 per 100,000** |
| | ~70% cardiac comorbidities and surgery, reduced life expectancy | ~50% cardiac comorbidities, reduced life expectancy; infertility | Short stature and skeletal deformities |

| Today with hGH | Potential Future with VOXZOGO |
|---|---|
| • Growth hormone approved to treat | • VOXZOGO approved to treat |
| • Diagnosis is often delayed | • Market development to enhance diagnostic testing |
| • Limited efficacy; waning in 70-80% children[27] | • Superior and sustained efficacy as seen in ACH |
| • Cardiac safety concerns[28] | • No major safety concerns |

*Total Addressable Patient Population eligibility includes those with height Z-score ≤ -2.5 SDs

(27) See appendix slide 74, reference 27
(28) See appendix slide 74, reference 28

# BMN 333: Potential Best-in-class Treatment for Skeletal Conditions

**Target Launch: 2030**

## Opportunity to Improve Upon VOXZOGO

Long-acting formulation would extend CNP franchise value

BMN 333 may offer improvement relative to VOXZOGO in multiple areas:

- Efficacy due to sustained exposure
- Safety confidence due to VOXZOGO's established profile
- Convenience due to less frequent dosing and presentation

Manufacturing efficiencies expected through improved formulation

**BMN 333**

**ACH & HCH**

## Market Strategy

Initial launch in ACH and HCH

Opportunity in new indications

## Initial Total Addressable Patient Population

ACH: ~24,000

HCH: ~14,000

# Meet Thinara, at 3 years old
(started VOXZOGO treatment at 6 months)

# Thinara at 5 years old

# Closing:

# BioMarin’s Differentiated and Compelling Thesis

**ALEXANDER HARDY**
President & Chief Executive Officer

# Transformational 2024: Delivering Strategic Changes & Strong Execution

## Implemented Significant Strategic Changes

- Revamped Corporate Strategy and Business Plan
- Unlocked plan to realize full potential value of VOXZOGO
- Prioritized pipeline, reflecting enhanced commercial lens
- Decided ROCTAVIAN future strategy earlier than anticipated
- New organizational structure & business cultural evolution
- New leadership team in place

## Operational Excellence and Discipline Driving 2024 Successes through Q2

- Business execution drove financial results exceeding expectations
- Achieving meaningful operating margin improvement earlier than expected
- Resolved VOXZOGO supply constraint earlier than anticipated
- Obtained FDA alignment on VOXZOGO expansion into five new indications

## We are Ready to Execute and Deliver More

- For patients
- For employees
- For shareholders

# BioMarin's Differentiated & Compelling Investment Thesis

## Top Quartile Biopharma Revenue Growth*

**TARGETING:**

~$4B Revenue in 2027

Mid-teen Revenue Growth through 2034

- **Skeletal Conditions: $5B+ revenue opportunity** across 6 indications
- **Enzyme Therapies:** targeting **high single-digit CAGR**

## Doubling Non-GAAP Operating Margin**

**TARGETING:**

40%+ Non-GAAP Operating Margin** Starting in 2026

- **$500M cost transformation**
- Leveraging **commercial presence in ~80 countries**

## Prioritized Innovation

**TARGETING:**

7 Indications in Phase 3 by 2027

11 Launches by 2034 including Two by 2027

- R&D Portfolio with **compelling commercial profile**
- **Industry-leading track record** (~90% approval of BMRN-sponsored pivotal programs in the last 20 years)

## Optimized Cash Flow & Capital Allocation

**TARGETING:**

$1.25B+ Operating Cash Flow in 2027

- **Substantial cash flow generation**, inclusive of organic R&D investments
- **Disciplined investment** in external assets to augment revenue growth with target deal size <$1.5B

*Refer to appendix slide 70 for BioMarin peer comparisons for top quartile revenue growth

**Refer to slide 3 and the appendix to this presentation for the definition of Non-GAAP Operating Margin. Reconciliation of forward-looking Non-GAAP Operating Margin to the most directly comparable U.S. GAAP reported financial measure is not available. Refer to the appendix slide 78 for further information regarding forward-looking Non-GAAP financial measures.

# Thank you.

**Q&A**


BIOMARIN

# Appendix I

## Guidance Assumptions, Peer Comparisons, Business Units Reference

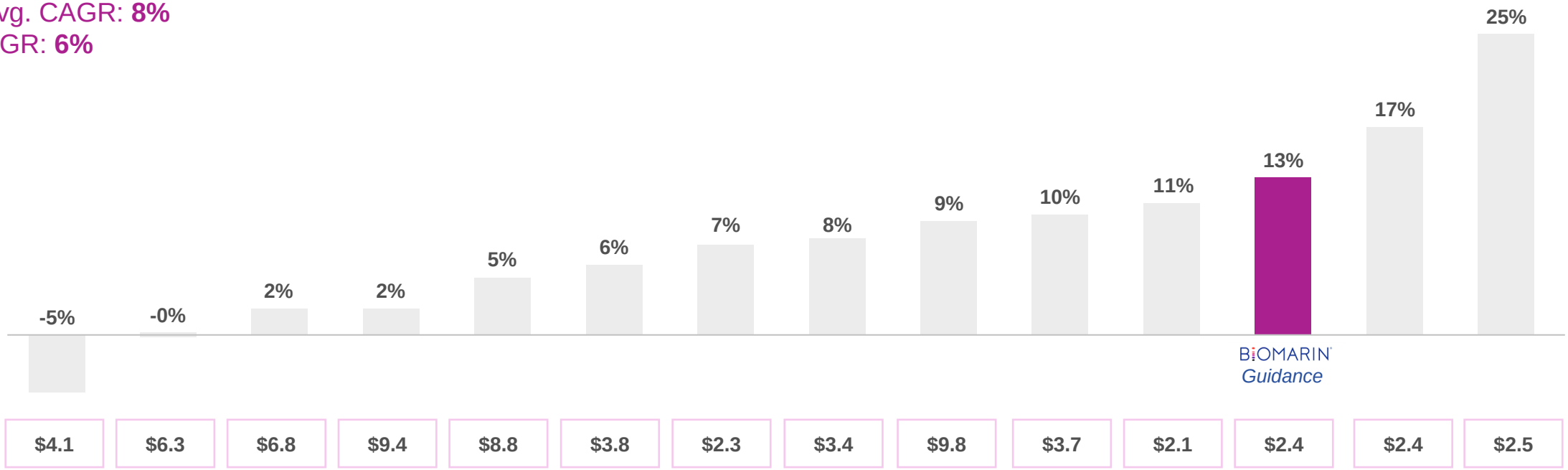
# Guidance Assumptions

- ☑ No Global Recession or Material BioMarin market instability
- ☑ No Major changes in US or Foreign policy
- ☑ Non-Risk Adjusted New Launch Revenues
- ☑ Non-GAAP P&L measures
- ☑ FX held at constant to current rates
- ☑ ~200M Diluted Shares
- ☑ No New External Innovation initiatives including potential divestitures or acquisitions

# BioMarin Peer Comparison: 2023-27 Revenue CAGR

## $2B - $10B Revenue Companies

Total Avg. CAGR: **8%**
WA CAGR: **6%**




Peer data as of 7/22/2024 FACTSET, BioMarin CAGR based on ~$4B '27 Guidance
Segmentation based on 2023 Revenue

# Business Units Reference

| BUSINESS UNIT | PROGRAMS INCLUDED |
|---|---|
| **Skeletal Conditions** | VOXZOGO for multiple indications:<br>• Achondroplasia<br>• Hypochondroplasia<br>• Idiopathic short stature<br>• Noonan Syndrome<br>• Turner Syndrome<br>• SHOX Deficiency<br>BMN 333 |
| **Enzyme Therapies** | VIMIZIM<br>NAGLAZYME<br>ALDURAZYME<br>BRINEURA<br>PALYNZIQ<br>BMN 390 |
| **ROCTAVIAN** | ROCTAVIAN |

# Appendix II

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# Appendix III

## Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

# Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information (in millions)

| | Twelve Months Ended December 31 | | | |
|---|---|---|---|---|
| | 2023 | Percent of GAAP Total Revenue (5) | 2021 | Percent of GAAP Total Revenue (5) |
| **GAAP Income from Operations** | **$ 158.1** | **6.5%** | **$ (82.3)** | **-4.5%** |
| Adjustments | | | | |
| Stock-based compensation expense | 207.1 | 8.6% | 197.3 | 10.7% |
| Amortization of intangible assets | 62.2 | 2.6% | 61.9 | 3.4% |
| Contingent consideration | - | 0.0% | 8.0 | 0.4% |
| Gain on sale of nonfinancial assets (1) | - | 0.0% | - | 0.0% |
| Severance and restructuring costs (2) | (0.5) | 0.0% | - | 0.0% |
| Loss on Investments (3) | 14.0 | 0.6% | - | 0.0% |
| FX Reclass (4) | 27.7 | 1.1% | 11.7 | 0.6% |
| Total Non-GAAP adjustments | 310.5 | 12.8% | 278.9 | 15.1% |
| Non-GAAP Income from Operations | $ 468.6 | 19.4% | $ 196.6 | 10.6% |

(1) Represents a payment triggered by a third party's attainment of a regulatory approval milestone related to previously sold intangible assets.

(2) These amounts were included in SG&A and represent severance and restructuring costs related to the company's 2024 portfolio strategy review and the associated organizational redesign efforts announced in the second quarter of 2024. These amounts also include impairments of certain right-of-use and fixed assets.

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

(4) In the first quarter of 2024, we changed our presentation of foreign currency transaction gains and losses resulting from remeasurement and idle plant costs within our Condensed Consolidated Statements of Comprehensive Income.

(5) Calculated based on 2023 and 2021 total revenues, which were $2,419 and $1,846 million, respectively.

# Appendix IV

## Forward-Looking Non-GAAP Financial Measures

# Forward-Looking Non-GAAP Financial Measures

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.