42nd Annual
J.P. Morgan Healthcare
Conference

BioMarin Pharmaceutical Inc.

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President and Chief Executive Officer

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Forward-Looking Statements

This non-confidential presentation contains "forward-looking statements" about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: the profitability opportunities and expected financial performance of BioMarin, including, without limitation, the expectation of significant multi-year growth driven by VOXZOGO, the projected label expansion of VOXZOGO and the pace of such expansion, VOXZOGO launch revenue trajectories, the ability to ensure VOXZOGO supply, the expected revenue growth potential of ROCTAVIAN, potential opportunities to increase profit margins and EPS, and other specified financial guidance; the markets for BioMarin's products and product candidates, including, without limitation, the addressable patient populations in BioMarin territories and commercial market opportunities; and BioMarin's development of product candidates and commercialization of products, including, without limitation, the opportunity to change the treatment paradigm for severe hemophilia A with ROCTAVIAN, the commercial launch of ROCTAVIAN, and the market opportunities for ROCTAVIAN. 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: the results of the review to be conducted by the Strategic and Operating Review Committee; the integration of the new directors on BioMarin's Board of Directors; results and timing of current and planned preclinical studies and clinical trials and the release of data from those trials; BioMarin's ability to successfully manufacture its commercial products and product candidates; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning products and product candidates; BioMarin's success in the commercialization of its commercial products; impacts of macroeconomic and other external factors on BioMarin's operations; 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, 2023 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.

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Agenda for Today

1. My Background and Relevant Experience
2. Why I Chose BioMarin
3. Key Priorities Ahead

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The Right Expertise to Drive Value Creation

A bit about me….

Former CEO of Genentech ($25 billion in revenue and 13,500 employees) **with over 30 years of industry experience** across global pharma industry and biotechnology

Track record of innovation, accelerating new product growth and delivering operational efficiencies

Lifecycle Management Experience

- Responsible for Global Product Strategy
- Led late-stage strategy, launch and LCM across oncology, benign hematology, neuroscience, immunology and ophthalmology

Commercial Excellence

- In last 5 years, launched 10 new medicines
- Returned company to strong growth following 3 simultaneous LOE events (~40% of revenue) and attained/sustained leadership positions in highly competitive markets

Operational Excellence

- Implemented operating model with novel ‘ecosystem approach’ and emphasis on omnichannel to drive superior customer experience and greater flexibility
- Delivered accelerating revenue growth, leveraged to the bottom-line

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Why I Chose BioMarin: At the Inflection Point of Opportunity

Strong Revenue Growth Potential	▪ Significant multi-year growth driver with VOXZOGO® in achondroplasia and new indications ▪ ROCTAVIAN™ for severe hemophilia A as a potential standard of care gene therapy ▪ Durable, profitable, "IRA-insulated" annuity-like base business
Significant Profitability Opportunities	▪ 8 approved products driving industry-leading revenue growth ▪ Financially self-sustaining as a profitable and cash flow positive enterprise ▪ Clear potential for greater EPS growth
Industry-leading Innovation through Proven R&D Engine	▪ 100% commercialization rate for Phase 3 assets; 8 internally developed commercial products ▪ >50% Investigational New Drug (IND) to marketing authorization approval rate ▪ Multi-modal expertise, global regulatory capabilities and wholly-owned manufacturing

BIOMARIN®

We Serve Markets with Innovative Medicines that Address Unmet Needs

5 Products Approved in the Last 9 Years

Enzyme Replacement Therapies

VIMIZIM® (elosulfase alfa)

Brineura® (cerliponase alfa)

ALDURAZYME® (LARONIDASE)

Naglazyme® (GALSULFASE)

~13,000
Patients benefitting from our therapies

Achondroplasia

VOXZOGO™ (vosoritide) for injection

Phenylketonuria

KUVAN® (sapropterin dihydrochloride)

Palynziq™ (pegvaliase) Injection

Hemophilia A Gene Therapy

ROCTAVIAN® (valoctocogene roxaparvovec) Solution for intravenous infusion

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VOXZOGO: Transforming the Lives of Children With Achondroplasia

Eve

Age 3, started VOXZOGO at age 1.5
on therapy for 1.5+ years

Label Expansion For An Earlier Start

Annika

At age 14, started VOXZOGO at age 8
on therapy for 6+ years

Long-Term Efficacy And Safety

Kennedy Kaye, “KK”

Age 10, started VOXZOGO at age 8
on therapy for 2+ years

Impact On Quality of Life

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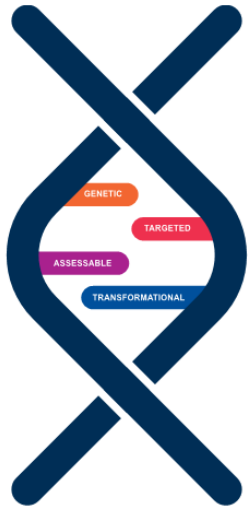
BioMarin's R&D Engine Remains Focused on Best-in-Class and/or First-in-Class Assets – *Prioritizing Candidates to be Accelerated*

Linking our Foundational "Core Four" to the Highest Potential Commercial Medicines

Leveraging genetic discoveries and tools, BioMarin has a clear understanding of the underlying disease mechanism

Study designs use readily assessable biomarkers/ endpoints that yield clear efficacy signals and reliably translate into clinical benefit



BioMarin can develop a targeted therapy that directly or proximally addresses the fundamental defect of the disease

The medicine has a transformational impact on patients' lives by profoundly improving the way they feel, function, and survive

TRANSLATING GENETIC DISCOVERIES INTO TRANSFORMATIVE MEDICINES

Early-stage Pipeline Optionality:

- 7 candidates in clinical/IND-enabling development
- Dozens of assets in research
- Multiple modalities and therapeutic areas

Thoughts on R&D Framework:

- Focus on assets with highest patient impact, significant market potential, and Return on Investment
- Disciplined spend through early development and high-bars for success
- Accelerate chosen highest value assets to maximize R&D investment productivity

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Thoughts on Initial Priorities – *Observations in First 40 Days*

ACCELERATE AND MAXIMIZE THE VOXZOGO OPPORTUNITY	▪ VOXZOGO indication expansion is **the** top priority for BioMarin ▪ Maintain momentum on achondroplasia launch and leverage broadened labels
ESTABLISH ROCTAVIAN OPPORTUNITY	▪ Important progress made in market access and readiness ▪ 2024 and 2025 will further inform ROCTAVIAN's uptake curve and long-term potential
FOCUS R&D ON THE MOST PRODUCTIVE ASSETS	▪ Prioritize therapies with transformational benefits and high commercial potential ▪ Efficient investment through proof-of-concept with stringent criteria before advancing assets
ACCELERATE EPS GROWTH AND EXPAND MARGINS	▪ Continued significant revenue growth, intense focus on operational excellence, prioritization of investments, and optimization of cost structure to accelerate the growth of profit margins and EPS

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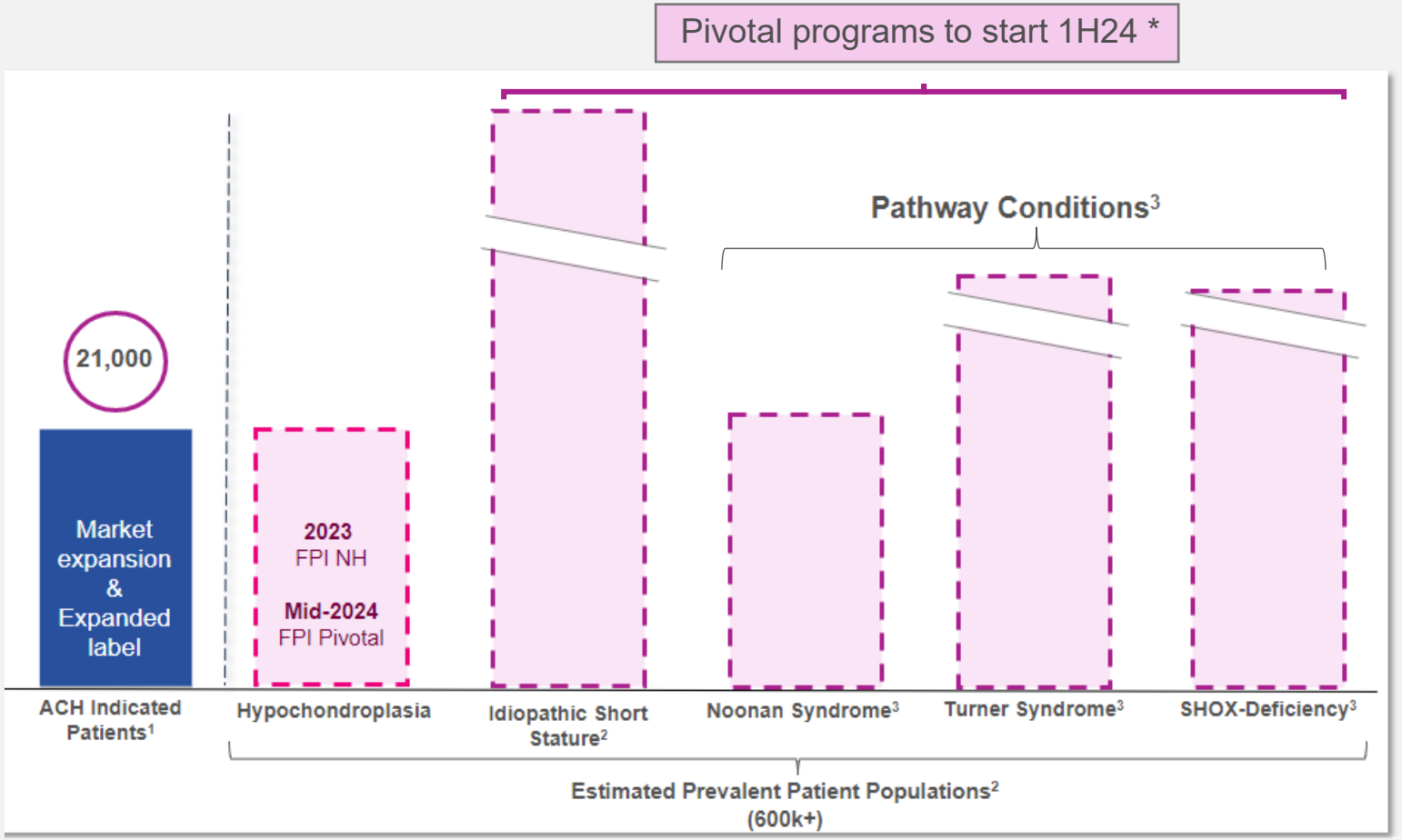
VOXZOGO: Opportunity to Expand in Achondroplasia (ACH) and Beyond

Leveraging more than 1000 patient years of experience in achondroplasia to accelerate new indications

- **Label expansion has increased HCP intent** to prescribe across all ages, including infants and toddlers
- **Ped Endos are taking ownership** of VOXZOGO and coordinating multi-disciplinary care
- **Peds more likely to refer patients** to Ped Endos, though also expect to increase prescribing
- **HCPs want to learn more** so they feel more comfortable initiating a treatment conversation
- **Genetic studies/investigator sponsored data** give us confidence that VOXZOGO will be an effective treatment for patients with severe growth disorders



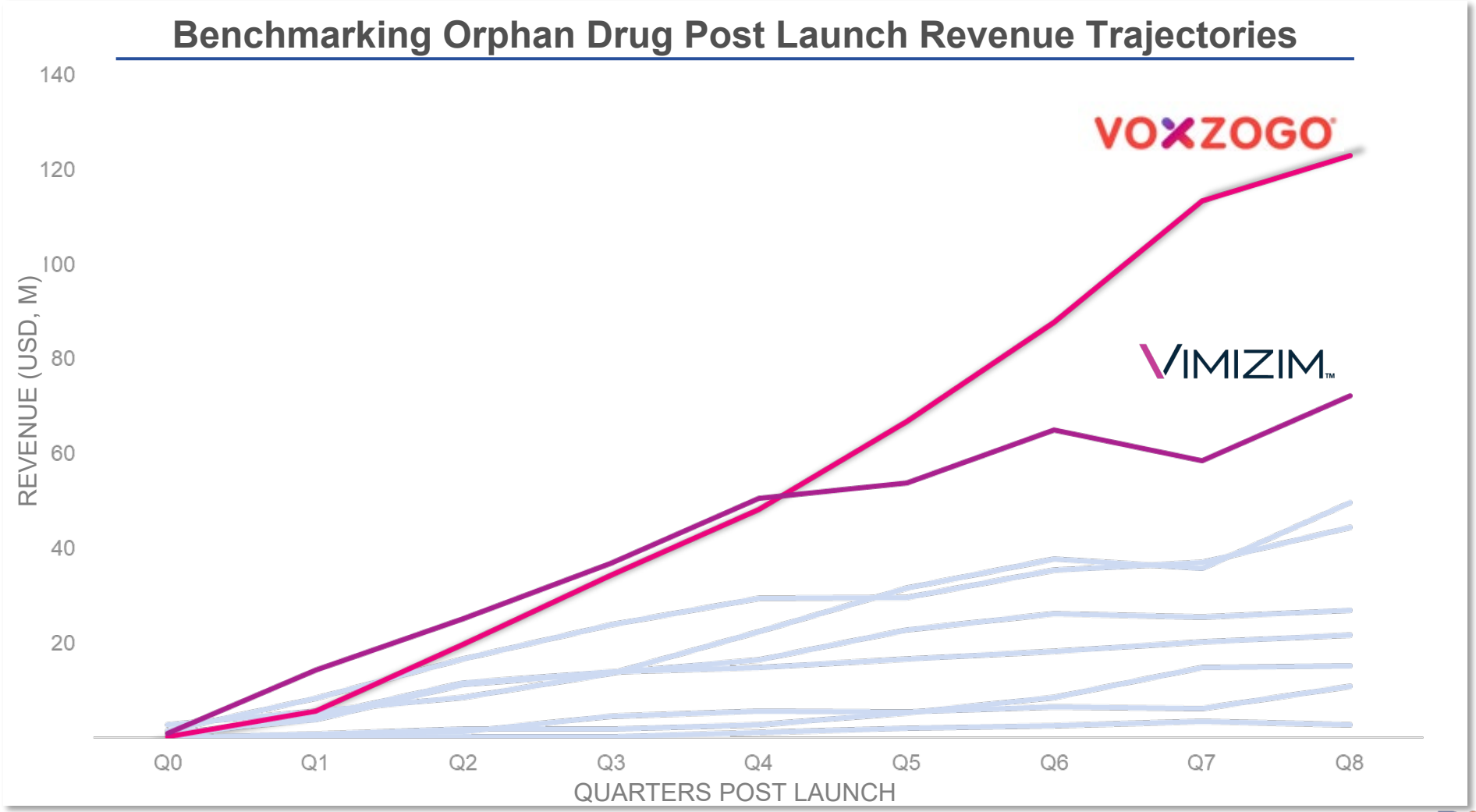
Source: Adapted from BioMarin Commissioned Global HCP Market Research (2023)

[1] Diagnosed prevalent patient population aged 0 – 15 within BioMarin's territories; [2] Estimated prevalent patient populations aged 0-15 across indications within BioMarin's territories (Idiopathic Short Stature indexes to patients with height z-score ≤-2.5 SD in target markets with human growth hormone approvals (U.S., Brazil)); [3] Pathway Conditions with height z-score <-2.0 SD

* Observational run-in studies in 1H24, in parallel seeking alignment with Health Authorities (HA) on interventional trials with treatment studies to initiate after final HA alignment

VOXZOGO in ACH: Strong Global Launch Execution and Rapid Uptake

VOXZOGO's commercial launch has outperformed numerous rare disease analogs



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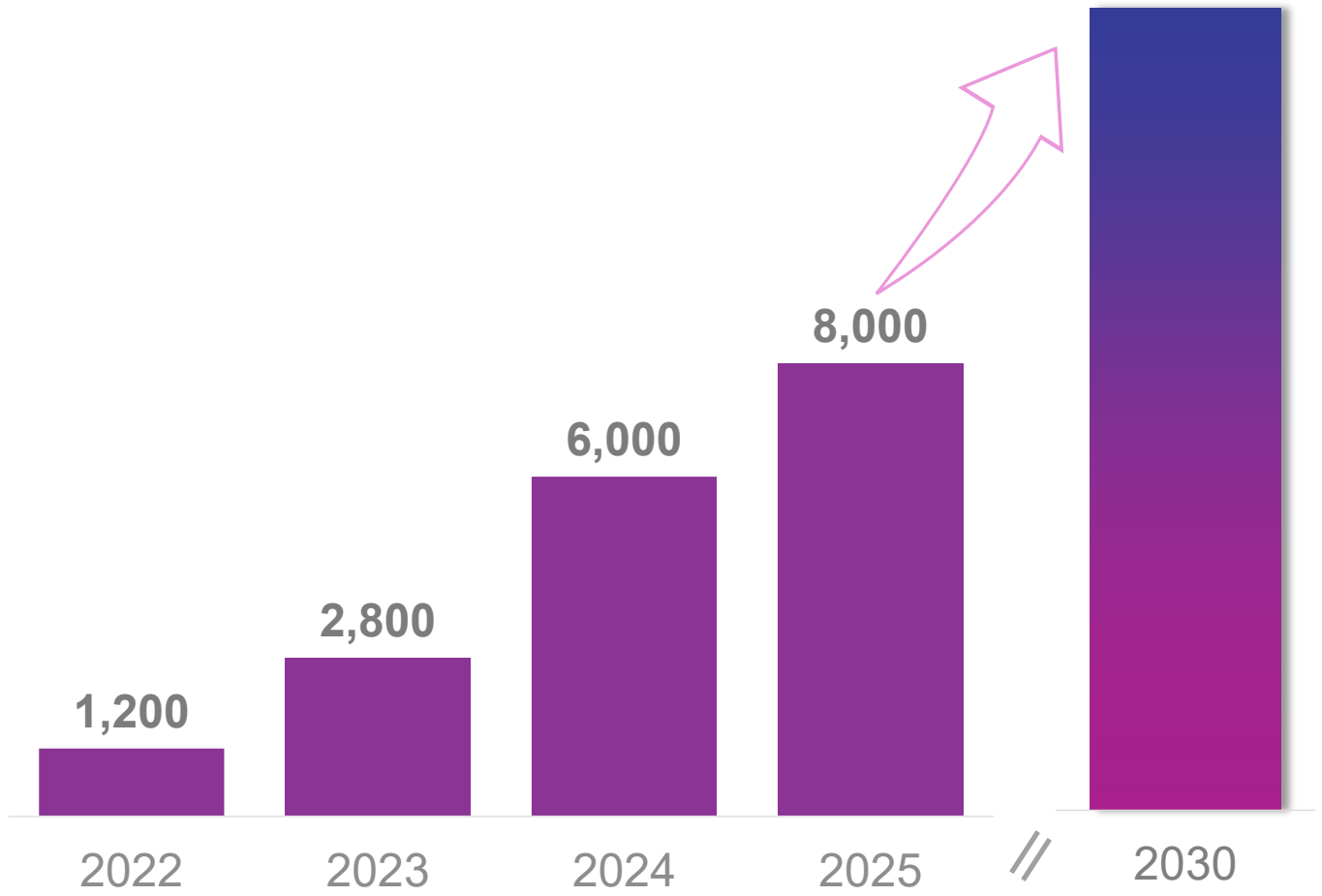
VOXZOGO: Managing Supply to Support Acceleration of Growth

Capacity to Serve VOXZOGO Patients



Global VOXZOGO Supply Capacity in Number of Patients

Ensuring VOXZOGO Supply

1. Secured additional near-term fill/finish capacity with CMO to expand VOXZOGO supply
2. Plan for fully unconstrained supply by mid-2024
3. Continued capacity expansion to supply long-term growth

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ROCTAVIAN: Value Proposition Remains Strong – Ramp Taking Time

Durable, One-time Treatment Offering Superior Outcomes Versus Standard of Care

- Significant reduction in treated bleeds compared with baseline
- Substantial reduction in FVIII usage compared with baseline
- Improvements in QoL
- Ability to be unburdened by prophylaxis for years

Cost Effective Solution

- Single infusion
- Novel Warranty Structure in the U.S.
- Favorable Review by Institute for Clinical and Economic Review (ICER)

Global Commercialization Strategy Utilizing Existing Global Infrastructure

- Global registrations and launch activities under way
- Essential U.S. & Germany pricing and reimbursement milestones achieved

Targeted Marketing Strategy

- Strategy to build market access network and be competitive
- Early market demand signals positive - Pioneering nature of launch with less urgency in the market is taking time

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Evolution of our Communications Approach in 2024 and Beyond

Maturing Guided Line Items
(4Q23 Earnings Call)

- Total Revenues
- Non-GAAP Operating Margin
- Earnings Per Share

More Consistent Cadence and Content of Updates

- Communicate business updates and objectives each quarter
- Focus on material developments and catalysts
- Scientific data presented at medical conferences and congresses

Investor Day 2024
(3Q24)

- New leadership perspectives on company strategy and long-term aspirations
- Strategic and Operating Review Committee outcomes
- Long-term financial targets

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Summary

1. Accelerate VOXZOGO Expansion
2. Execute on ROCTAVIAN
3. Prioritize R&D Assets
4. Increase Profitability Faster

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Thank You

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Transforming Lives Through Genetic Discovery