Barclays Global Healthcare Conference

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Brian Mueller

Executive Vice President, Chief Financial Officer



BIOMARIN®

Transforming Lives Through Genetic Discovery

Forward-Looking Statements

This non-confidential presentation contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements regarding expectations of growth and profitability, expectations for results of operations and financial guidance for the full-year 2023 and BioMarin's clinical development and commercial prospects, including, without limitation, (i) the duration and outcome of the FDA's review of BioMarin's BLA resubmission for ROCTAVIAN and (ii) potential market expansion opportunities for VOXZOGO and 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, 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 Annual Report on Form 10-K for the year ended December 31, 2022 as such factors may be updated by any subsequent reports. You 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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Non-GAAP Financial Measures

This presentation includes both GAAP information and Non-GAAP information. For BioMarin's 2023 financial guidance, Non-GAAP Income is defined as GAAP Net Income excluding amortization of intangible assets, stock-based compensation expense, and certain other specified items. The Company also recently introduced a new Non-GAAP measure, Non-GAAP Diluted EPS which is defined as Non-GAAP Income divided by Non-GAAP diluted shares outstanding.

Non-GAAP Income and its components 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.

The reclassified 2022 and 2021 non-GAAP information calculated consistent with the 2023 Non-GAAP Income methodology is available at Biomarin.com

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BioMarin is a Best-In-Class Genetic Disease Company At An Inflection Point of Transformative Growth

Solid, Profitable, “IRA-proof” & Growing Base Business

- Anchored by durable enzyme-based therapies producing nearly $2B of revenue and positive cash flows. Steady revenue growth across the portfolio
- High barriers to entry results in lower risk of competition and long-lived revenue streams

New Large-market Launches Are Significant Near-Term Growth Drivers

- VOXZOGO® as first treatment for Achondroplasia – BioMarin’s strongest global launch to date
- ROCTAVIAN™ poised to revolutionize treatment for Severe Hemophilia A with First Gene Therapy to launch in a large, chronically managed population with unmet medical needs
- Profit margin expansion expected as new product revenue growth leverages world class infrastructure

Best-In-Class Innovation Capabilities

- Industry-leading R&D teams produced suite of eight internally-developed genetic-based commercial therapies
- Fully-integrated and scaled biopharma capabilities: Discovery, Clinical, Regulatory and world-class Manufacturing
- Leveraging decades of genetic expertise in current age of rapid genetic disease discovery
- Exceptional development success rate applied to deep early-stage pipeline with most assets in Company history

Fourth Quarter and Full Year 2022 Key Business Highlights

BioMarin Achieves Record $2.1 Billion in Total Revenues for Full Year 2022
Full year sales increased 20%, excluding KUVAN®, and contribute to positive net income for the year

Rapid Patient Uptake and Commercial Execution Drives 2022 VOXZOGO® Sales of $169M
Expect revenue to more than double in 2023[1]; FDA accepted sNDA to expand use of VOXZOGO® to treat children with Achondroplasia <5

ROCTAVIAN™ EU Commercial Launch Underway
Patient testing to determine eligibility for ROCTAVIAN treatment ongoing throughout Germany

ROCTAVIAN™ 3-Year Phase 3 Results Submitted to FDA – Met all Efficacy Endpoints
U.S. Prescription Drug User Fee Act (PDUFA) target action date currently set for June 30, 2023

Expect 2023 Revenue Growth to Exceed 15%; Net Income Growth of Approximately 30%[1]
Driven by BioMarin's proven global commercial capabilities and commitment to sustainable profitability

[1] Based on mid-point of full year 2023 guidance issued February 27, 2023.

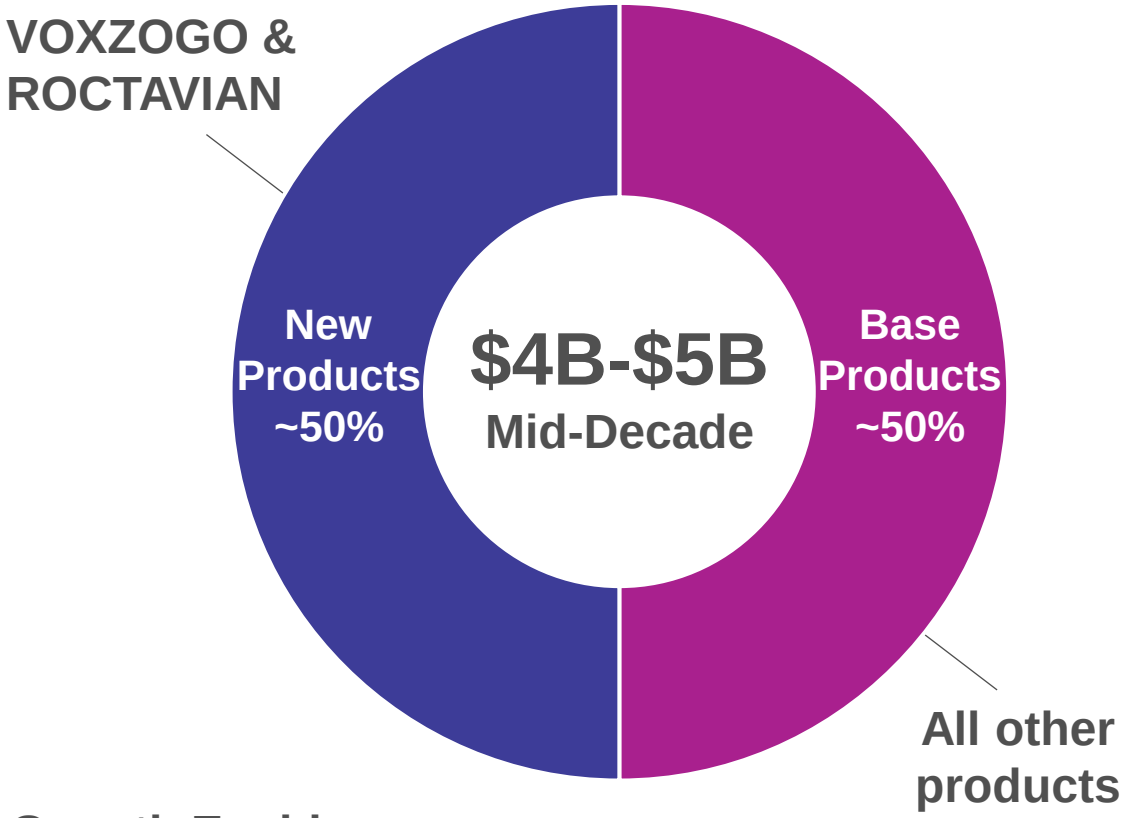
Full Year 2023 Guidance

(In millions, except per share and percentage data)	2022 Actuals	2023 Guidance[1]
Enzyme Products[2]	$1,645	$1,700 - $1,850
ROCTAVIAN™	--	$100 - $200
VOXZOGO®	$169	$330 - $380
Total Revenues	**$2,096**	**$2,375 - $2,500**
Gross Margin %	76.9%	77.5% - 79.0%
R&D % of Revenue	31%	30% - 32%
SG&A % of Revenue	41%	36% - 38%
GAAP Net Income	**$142**	**$155 - $205**
GAAP Diluted EPS	**$0.75**	**$0.78 - $1.03**
***New* Non-GAAP Net Income[3]**	**$291**	**$360 - $410**
***New* Non-GAAP Diluted EPS[4]**	**$1.53**	**$1.80 - $2.05**

[1] The full-year 2023 ROCTAVIAN guidance range provided above assumes a U.S. approval in 2023, regardless of approval timing.
[2] Enzyme Products include ALDURAZYME, VIMIZIM, NAGLAZYME, BRINEURA, and PALYNZIQ.
[3] Beginning in 2023, BioMarin defines Non-GAAP Income (Loss) as GAAP Net Income (Loss) excluding amortization of intangible assets, stock-based compensation expense, and certain other specified items. The reclassified 2022 and 2021 non-GAAP information calculated consistent with the 2023 Non-GAAP Income methodology is available at Biomarin.com
[4] 2023 GAAP and Non-GAAP Diluted EPS guidance assumes ~200 million shares.

Significant Future Growth Expected Driven by VOXZOGO and ROCTAVIAN





Anticipated Revenue Growth Enablers

Continued growth within base portfolio	**Base indication launch of VOXZOGO & ROCTAVIAN**	**Regulatory approval of U.S. ROCTAVIAN**	**Expansion of VOXZOGO & ROCTAVIAN indications**

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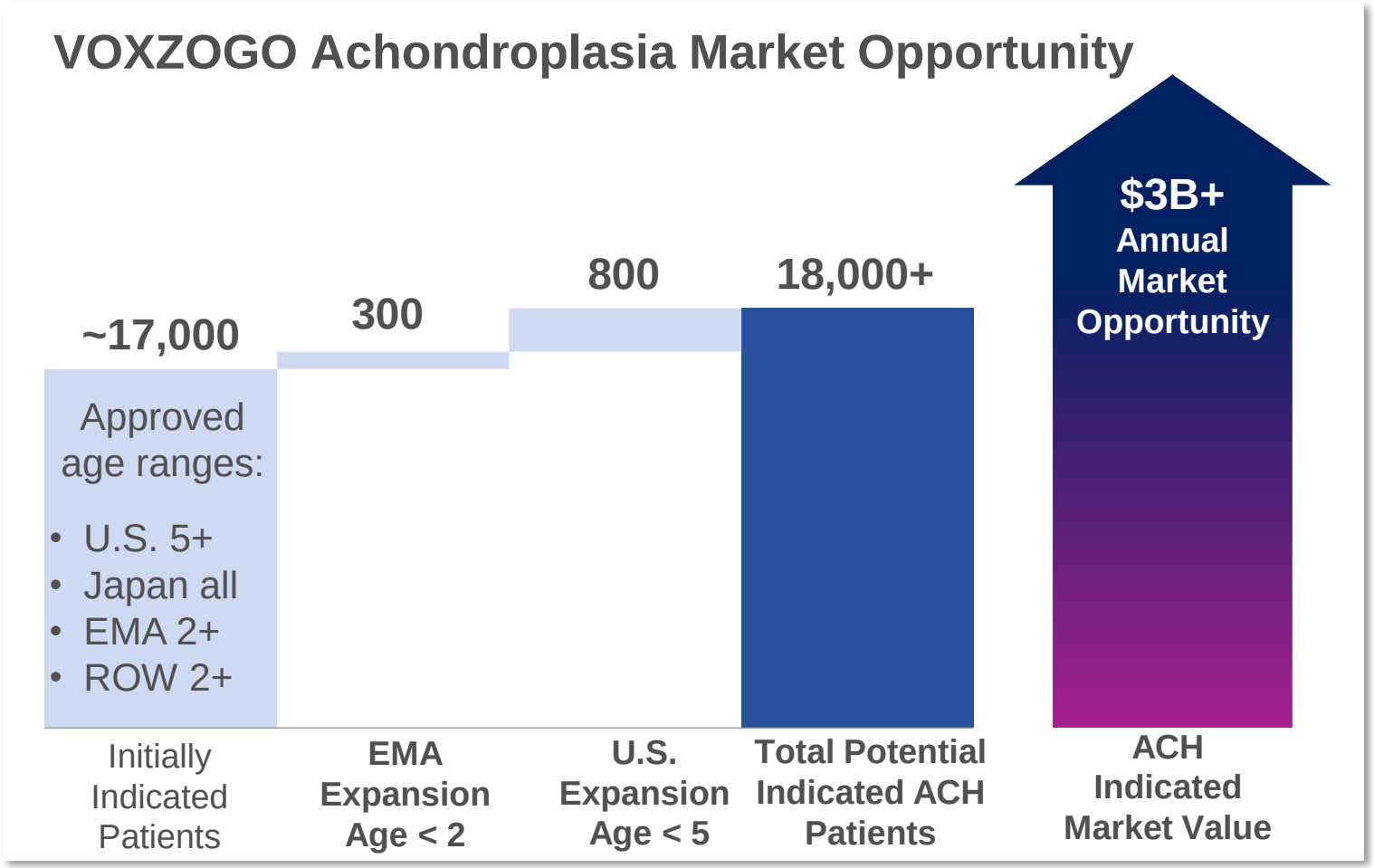
FDA Accepts sNDA to Expand Use of VOXZOGO® to <5 years of age

1,000+ Additional Children Eligible for Treatment upon Anticipated U.S. and E.U. Age Expansions



(Approximately 1,000 newborns with achondroplasia born each year in BioMarin territories)

Launch Excellence

2022 Commercial Execution

- 1 year anniversary
- Over **1,000** children treated at year-end
- 32 active markets to date
- $169M 2022 revenue

2023 Opportunities

- PDUFA target action date of October 2023 for < 5 y/o; EU 2H23 for <2 y/o
- Continued uptake of patients, and expansion into additional new markets

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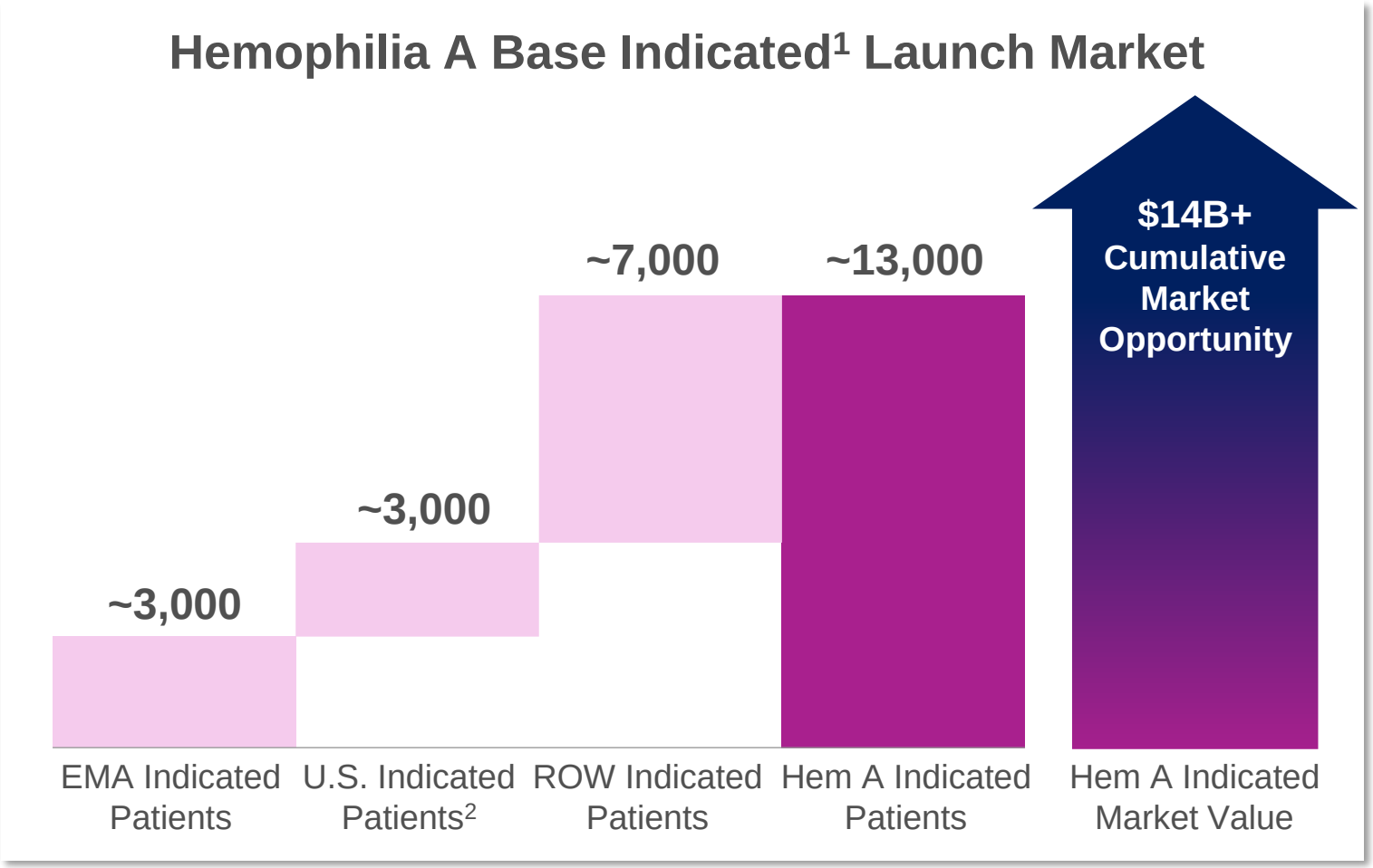
ROCTAVIAN: A Global $14B+ Cumulative Market Opportunity in Initial Indication



Strategic Market	Market Access Goal
Germany	1Q 2023
Italy	2H 2023
France	2H 2023
U.S.	2023
Japan	Mid-Decade
Brazil	Mid-Decade

[1] Indicated defined as consistent with Phase 3 eligibility criteria [2] U.S. ROCTAVIAN indication as anticipated 1H 2023

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U.S. ROCTAVIAN Commercial and Regulatory Progress

U.S. Market Access Plans Advancing:

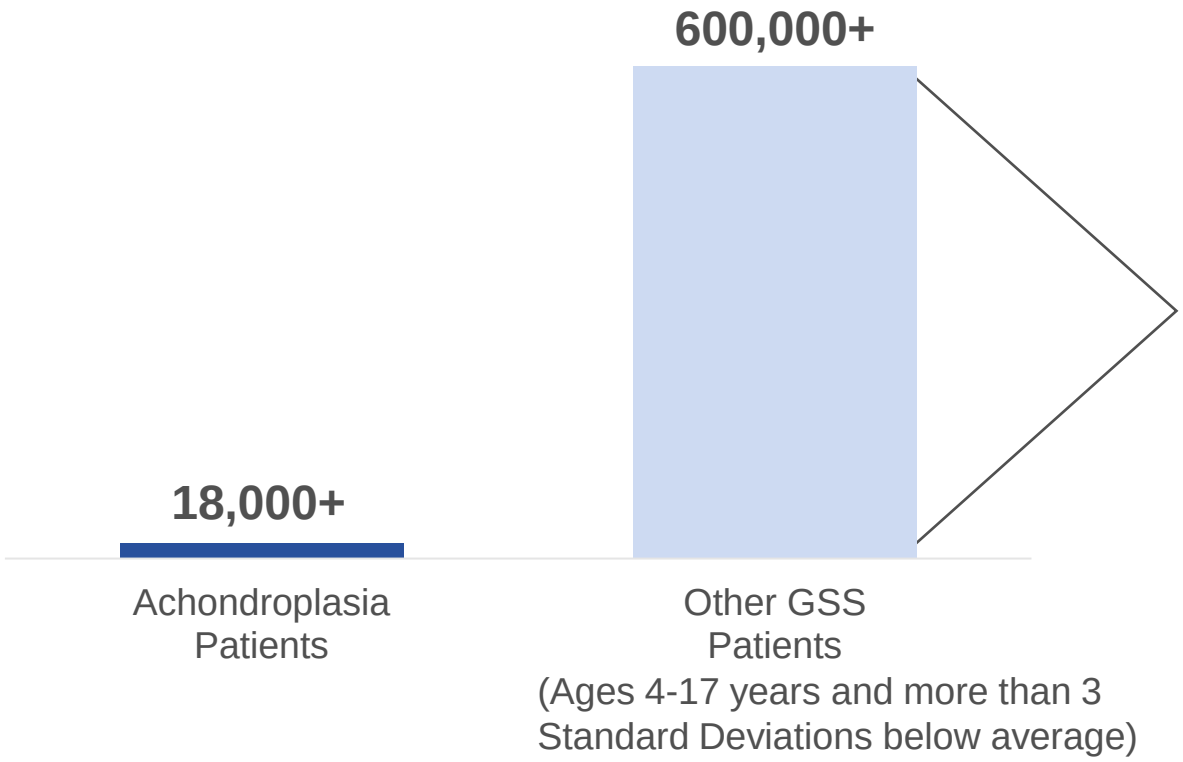
- ICER final assessment confirmed assumed value of ROCTAVIAN at $2.5M
- Approached by multiple significant U.S. insurers indicating intent to implement a favorable policy for ROCTAVIAN reimbursement
- U.S. outcomes-based agreements will be offered in the form of a warranty

U.S. Regulatory Progress:

- FDA completed the Pre-License inspection of our Gene Therapy facility in December; Believe the agency's comments and observations are addressable and are preparing for launch
- Clinical site inspections ongoing, with one more expected in March
- Submitted 3-year Phase 3 results to FDA; PDUFA extended target action date to June 30, 2023

Multi-Billion Dollar Global Market Opportunity with VOXZOGO Beyond Achondroplasia

VOXZOGO / CNP-Mechanism: Other Genetic Short Stature (GSS) Conditions



GSS conditions currently in the clinic:

- Hypochondroplasia
- CNP Deficiency
- Heterozygous NPR2 mutation
- Rasopathy
- SHOX
- Aggrecan Deficiency

Phase 2 Results and Next Clinical & Regulatory Steps Expected Mid-2023

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Advancing the Most Diverse and High-potential Pipeline in Our History

Updates coming at R&D Day September 12, 2023 in New York

	Therapeutic Focus	Product Candidate	Research	IND-Enabling	Phase 1	Phase 2
Research and Early Development	Musculoskeletal/ Metabolic	**BMN 255 Hyperoxaluria** (Small Molecule)				
		BMN 351 DMD (Exon 51 Oligonucleotide)				
		BMN 349 A1ATD (Small Molecule)				
		Long Acting CNP (Peptide)				
		MSK (Oligonucleotide)	3 Candidates			
		MSK (Gene Therapy)				
	Non-Oncology Hematology	**HEM** (Biologic)				
		HEM (Oligonucleotide)				
	Cardiovascular	**BMN 331 HAE** (AAV Gene Therapy)				
		BMN 293 MYBPC3 HCM (AAV Gene Therapy)				
		CV (AAV Gene Therapy)	4 Candidates			
		CV mAB				
	Central Nervous System	**CNS AAV** (Gene Therapy)	4 Candidates			
		CNS (Oligonucleotide)	4 Candidates			
		CNS (Peptide)				
		CNS (Biologic)				
	Total Addressable Market For IND Enabling & Phase I Programs			***$30B+***		

Creating Markets that Address Unmet Needs

5 Products Approved in the Last 8 Years

Enzyme Replacement Therapies

VIMIZIM® (elosulfase alfa)

Brineura® (cerliponase alfa)

ALDURAZYME® (LARONIDASE)

Naglazyme® (GALSULFASE)

Achondroplasia

VOXZOGO™ (vosoritide) for injection



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Phenylketonuria

KUVAN® (sapropterin dihydrochloride)

Palynziq™ (pegvaliase) Injection

Hemophilia A Gene Therapy

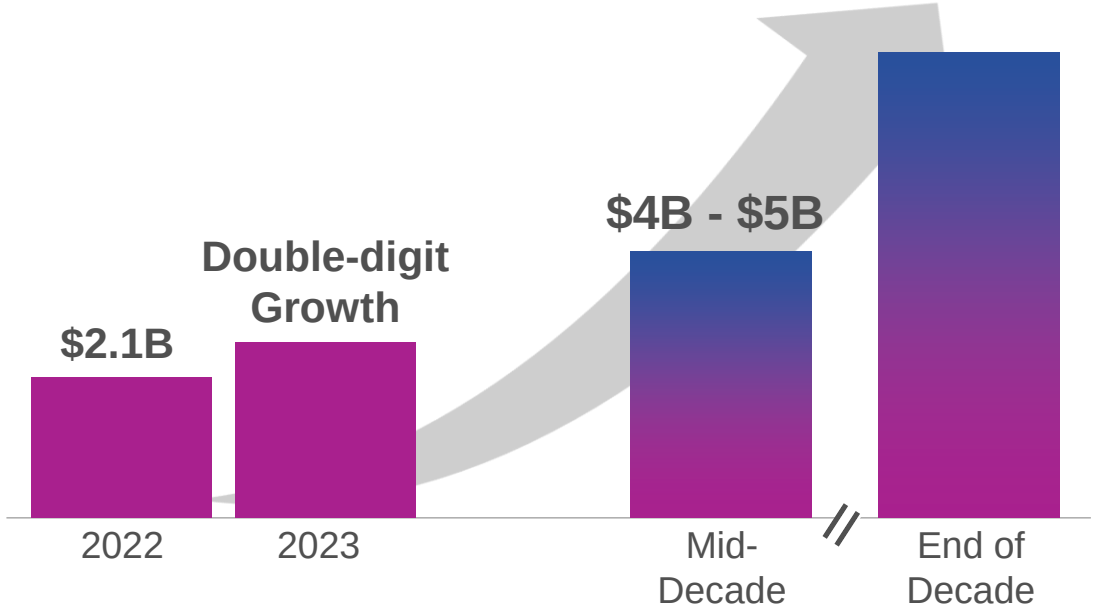
ROCTAVIAN® (valoctocogene roxaparvovec) Solution for intravenous infusion

Accelerating Revenue and Profitability Through Innovation

Near Term Revenue Growth Enablers

- Launch VOXZOGO and ROCTAVIAN base indications
- Achieve regulatory approval for U.S. ROCTAVIAN
- Expand VOXZOGO and ROCTAVIAN indications
- Continue to grow base portfolio

Accelerating Revenue Through Mid-Decade & Beyond



Financial Expectations

$4B - $5B of Revenue in Mid-Decade	Continuing to Invest in R&D Pipeline Advancements	Committing to Large BioPharma Peer Profit Margins

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Well Positioned to Deliver Sustainable Growth and Value Creation

Maximize Base Indication Revenue	• Durable base business and launch of two potential blockbuster products drive opportunity to double revenue by mid-decade
Aggressive Life Cycle Management	• VOXZOGO beyond Achondroplasia opportunity significantly out-sizes base indication • Studies underway to pursue ROCTAVIAN indications that more than double the base indication
Accelerate Clinical Pipeline	• Proven track record applied to the most early-stage development programs in Company history expected to drive revenue growth throughout decade and beyond
Balance Profits With Reinvestment	• Plans for sustainable profitability growth and cash accumulation to enable a sound capital allocation strategy focused on maximizing shareholder return

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Reconciliation of GAAP Net Income to Non-GAAP Income and Dilutive EPS Guidance

(In millions, except per share data)

	2023 Guidance[1]		
	Net Income	Diluted Shares	Diluted EPS
GAAP Net Income & Diluted EPS	$155 to $205	200	$0.78 to $1.03
Amortization expense	60		$0.30
Stock-based compensation expense	207		$1.04
Tax effect of adjustments[2]	(62)		($0.32)
Non-GAAP Income & Diluted EPS	$360 to $410	200	$1.80 to $2.05

*Totals may not foot due to rounding

[1] The adjustments/reconciling items included in the Guidance Year Ending December 31, 2023 column are presented to facilitate the reconciliation of Non-GAAP Income to its closest GAAP financial metric, GAAP Net Income. The Company notes that the specific amounts included in each reconciling line item above represent approximations of the underlying adjustments from GAAP Net Income to Non-GAAP Income, and that actual 2023 results for each reconciling line item may be different, in some cases materially, than the amounts listed above as a result of uncertainty regarding, and the potential variability of, those items.

[2] Income tax adjustments represent the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable statutory income tax rate.

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Thank You!

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