41st Annual J.P. Morgan Healthcare Conference

January 9, 2023

J.J. Bienaimé

Chairman and Chief Executive Officer



BIOMARIN®

Transforming Lives Through Genetic Discovery

Safe Harbor

This non-confidential presentation contains “forward-looking statements” about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: the expected financial performance of BioMarin, including, without limitation, preliminary unaudited total revenues and revenue from sales of VOXZOGO for fiscal year 2022, double-digit revenue growth in 2023, sustainable growth and profitability, profit margin expansion, mid-decade revenues, 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, IND submissions anticipated in 2023, the outcome of BioMarin's BLA resubmission for ROCTAVIAN to the FDA, and the commercial launch of 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: completion of BioMarin’s normal quarter and year-end accounting procedures for the quarter and the year ended December 31, 2022 and completion of the external audit with respect to such periods by BioMarin’s independent registered public accounting firm; results and timing of current and planned preclinical studies and clinical trials; BioMarin’s ability to successfully manufacture its commercial products and product candidates; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities concerning products and product candidates; BioMarin’s success in the commercialization of its commercial products; and those factors detailed in the press release issued January 8, 2023, as well as 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, 2022 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.

BIOMARIN

Scientific Breakthroughs are Foundational to Our Performance



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

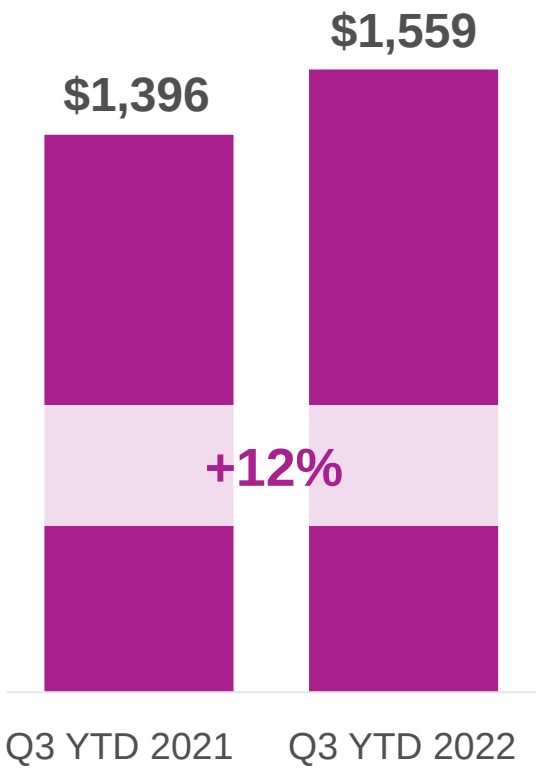
Best-In-Class Innovation Capabilities

- 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

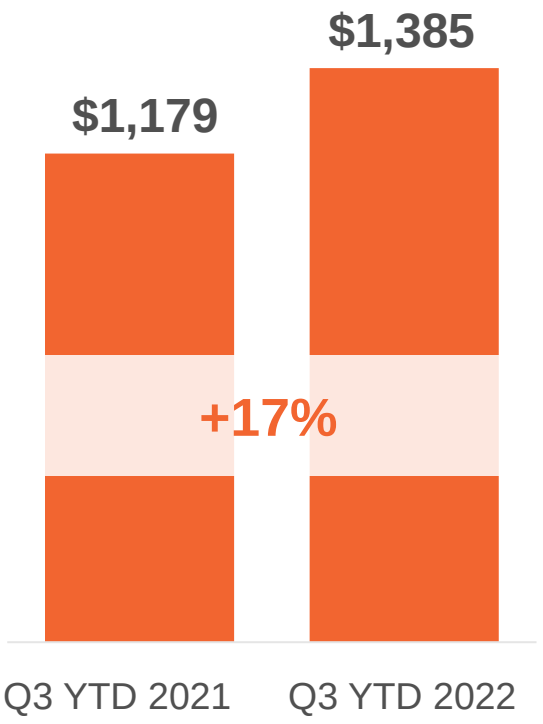
Third Quarter Year-to-Date: Double Digit Revenue Growth with Pivot to Sustainable GAAP Profitability Growth

$ Millions

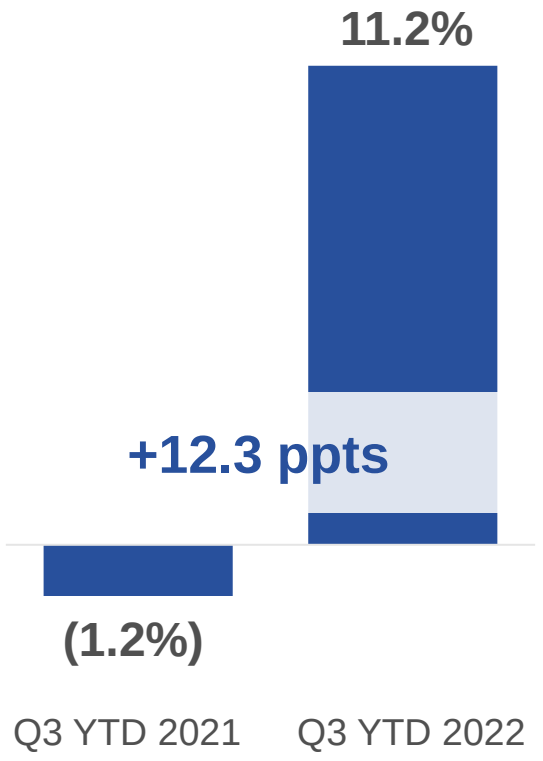
Reported Revenue Growth



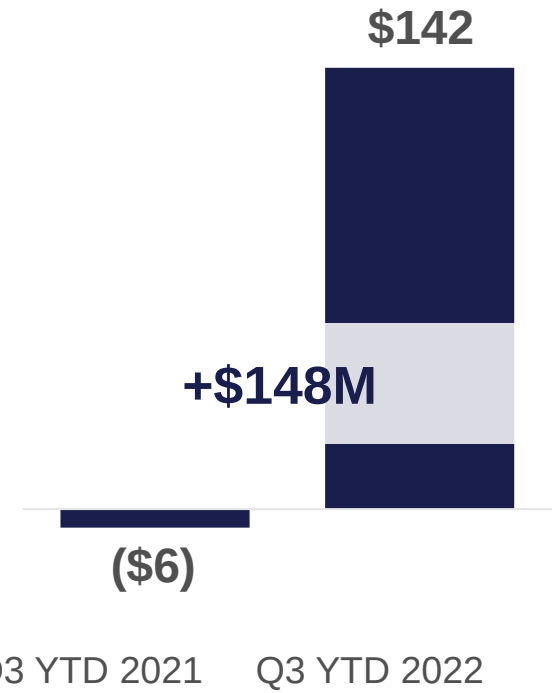
Revenue Growth Excluding Kuvan®



Operating Margin Expansion[1]

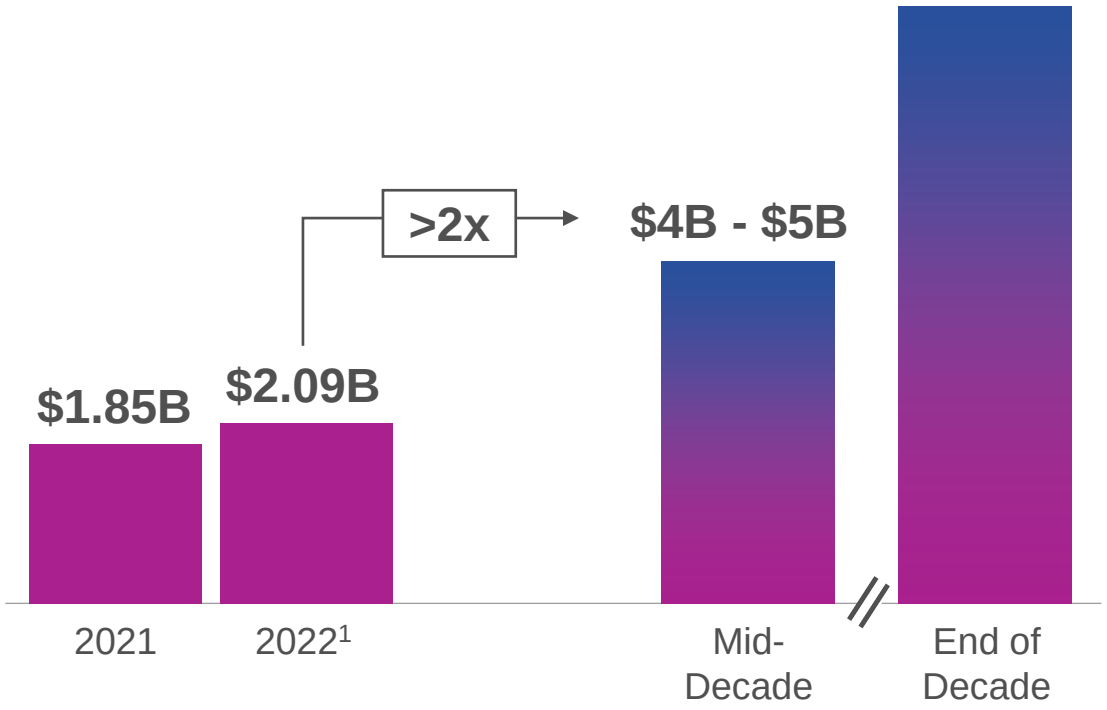


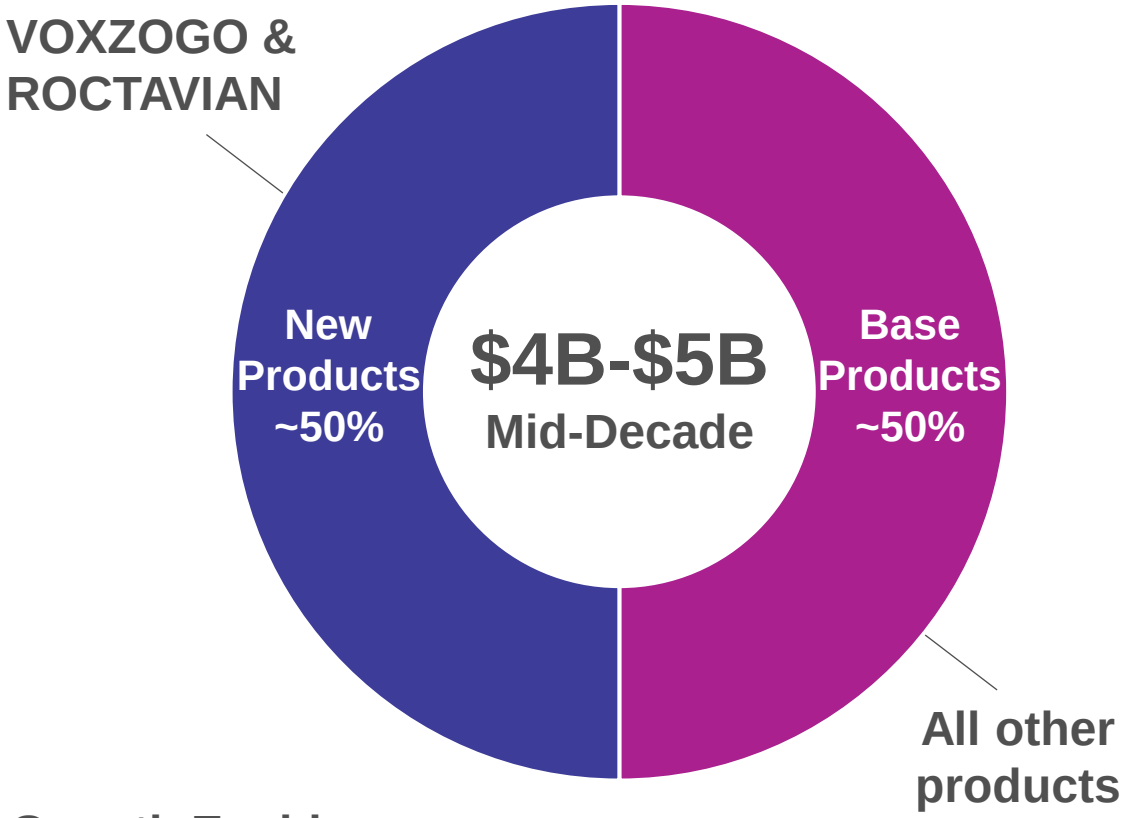
GAAP Net Income[1]



[1] Operating Margin Expansion and GAAP net income in 2022 includes $108M one-time pretax benefit from sale of priority review voucher (PRV); "YTD" in all instances denotes results through Q3

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**

[1] Unaudited preliminary 2022 revenue estimate

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Our Strategic Priorities To Drive Sustainable Growth & Value Creation

Maximize Base Indication Revenue

Aggressive Life Cycle Management

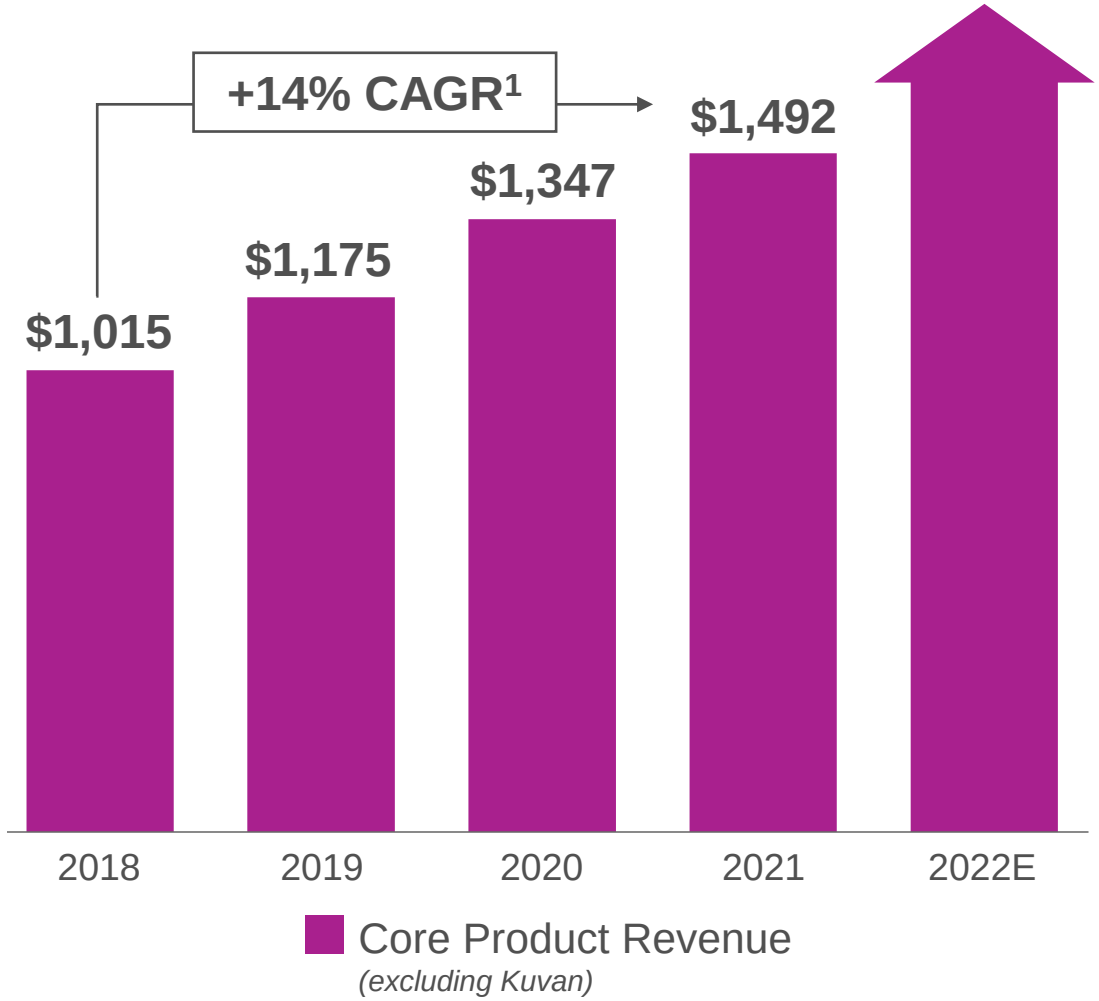
Sustainable Growth & Profitability

Accelerate Clinical Pipeline

Balance Profits with Reinvestments

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Core Products Underpin Sustainable Growth and Profitability



$ Millions

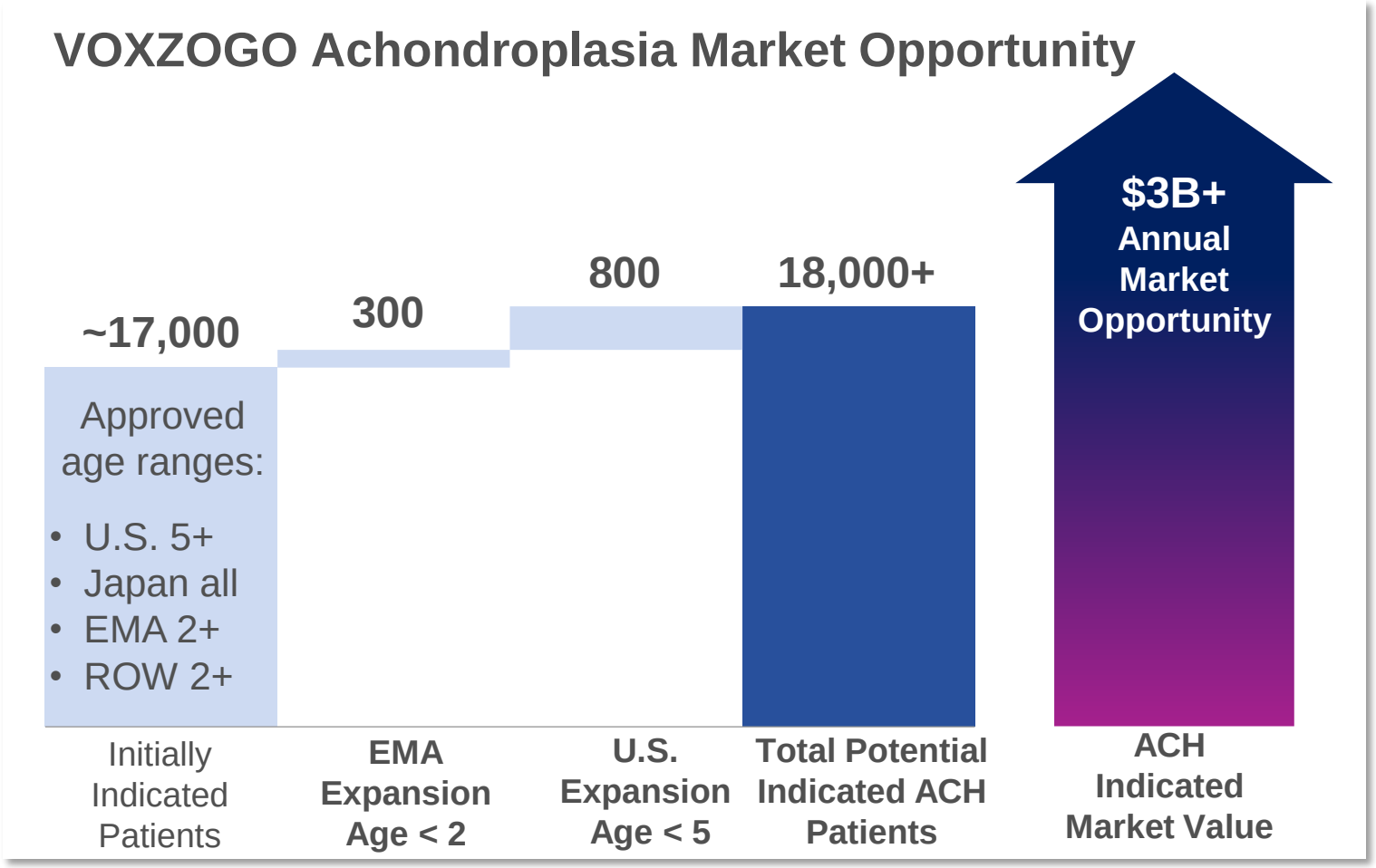
Product	2021	CAGR[1] 2018-21
VIMIZIM®	$623	+9%
NAGLAZYME®	$380	+3%
PALYNZIQ®	$237	+169%
BRINEURA®	$128	+48%
ALDURAZYME®	$123	(3%)
Core Products *(excluding Kuvan)*	**$1,492**	**+14%**

[1] Compounded annual growth rate 2018-21

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VOXZOGO: Opportunity to Expand Markets in Achondroplasia (ACH)

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[1] expected 2022 revenue

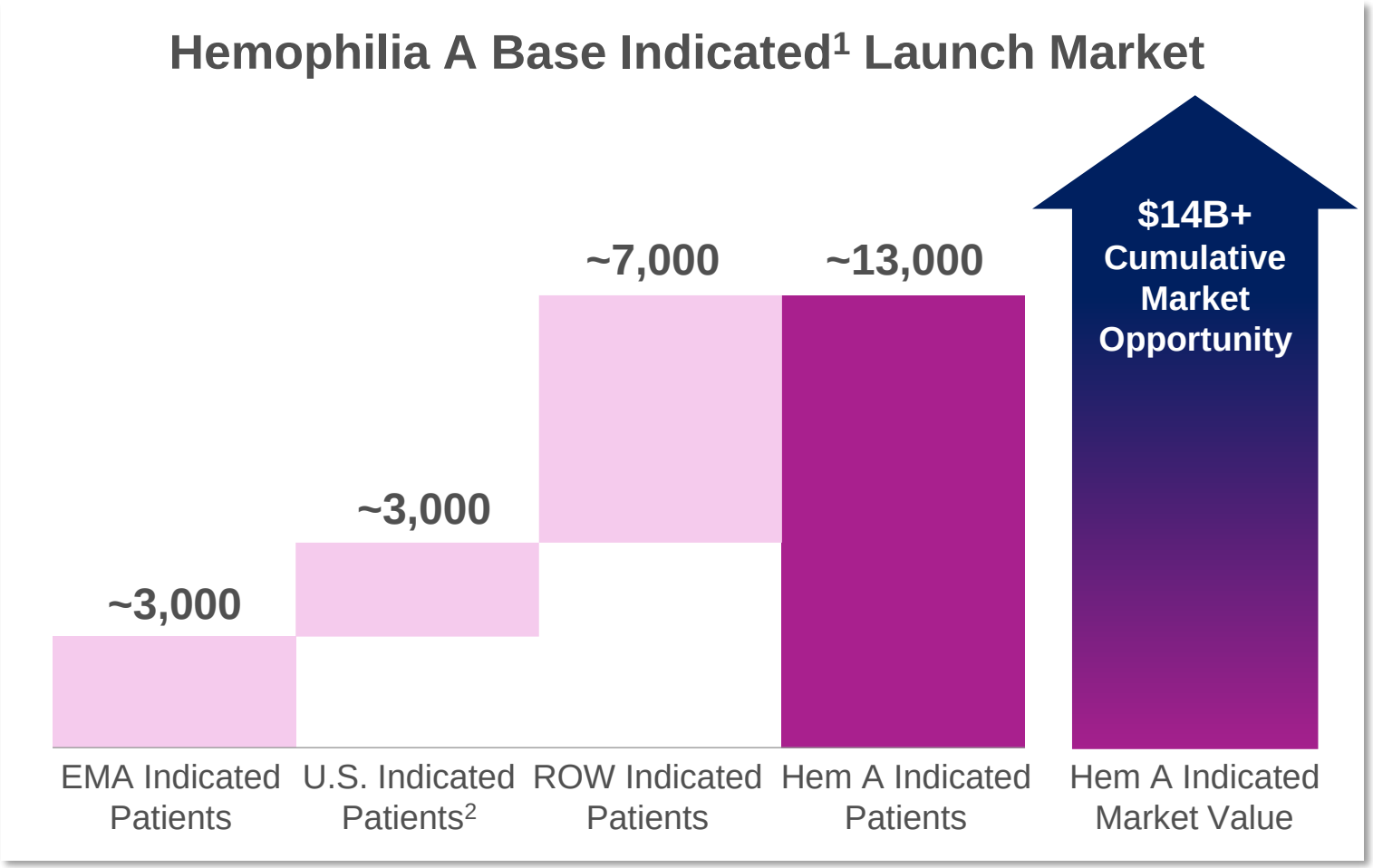
2023 Opportunities

- Continued uptake of patients, and expansion into additional new markets
- Potential age label expansions in the U.S. and Europe

[1] Unaudited preliminary 2022 revenue estimate

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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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The Transformational Impact of ROCTAVIAN on Patients Living with Severe Hemophilia A

“The impact of gene therapy on my day to day, it has been a life change, it has been a big change...**The peace of mind of living day to day, without being afraid of everything. That's the 3 years that I've had so far. A normal life.”**

“No bleeding, not even once.”



ROCTAVIAN Clinical Trial Patient

ROCTAVIAN E.U. Commercial Outlook Tracking to Plan

Outcomes Based Agreements (OBAs)	2023 Outlook
• First OBA concluded recently representing multiple German health insurers • Additional completed OBAs in Germany anticipated in the coming weeks • Patient CDx testing underway to determine ROCTAVIAN eligibility	• First German patients expected to be treated in the coming weeks • Comfortable with ROCTAVIAN full-year 2023 consensus estimates, assuming March U.S. approval • Full-year 2023 ROCTAVIAN revenue guidance to be provided in February

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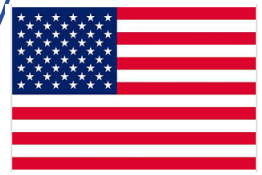
Strong Interest from Health Care Providers (HCPs) in EMA and US for ROCTAVIAN Treatment





~80% of HCPs likely to adopt ROCTAVIAN within 1 year of availability

ROCTAVIAN could capture ~35% of severe adult hemophilia A patients, *per EU HCPs*



~75% of HCPs likely to adopt ROCTAVIAN within 1 year of availability

ROCTAVIAN could capture ~40% of severe adult hemophilia A patients, *per US HCPs*

Source: BioMarin commissioned market research; Hemophilia A: Global 301 2+ Year Data Impact Assessment, EU (Germany, France, Italy) and US; April 2022

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 by FDA scheduled
- 3-year Phase 3 results to be submitted to FDA in the coming weeks

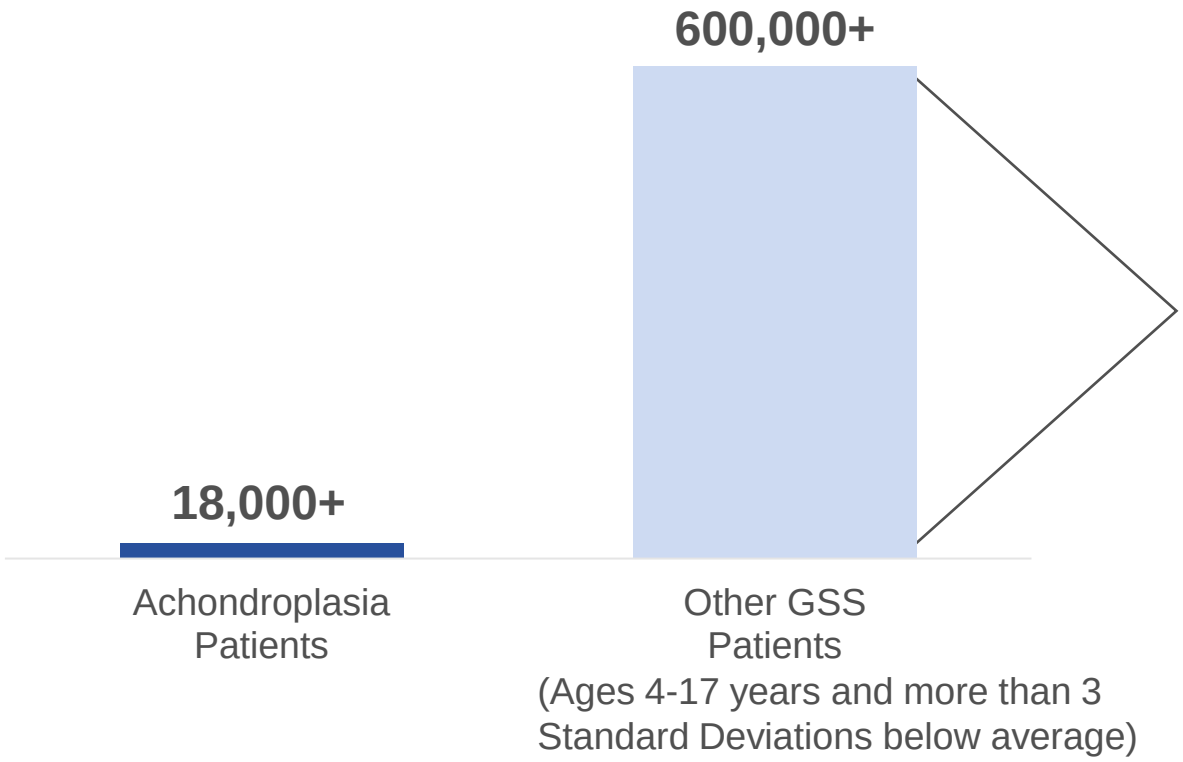
Our Strategic Priorities To Drive Sustainable Growth & Value Creation



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Multi-Billion Dollar Global Market Opportunity with VOXZOGO Beyond Achondroplasia

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



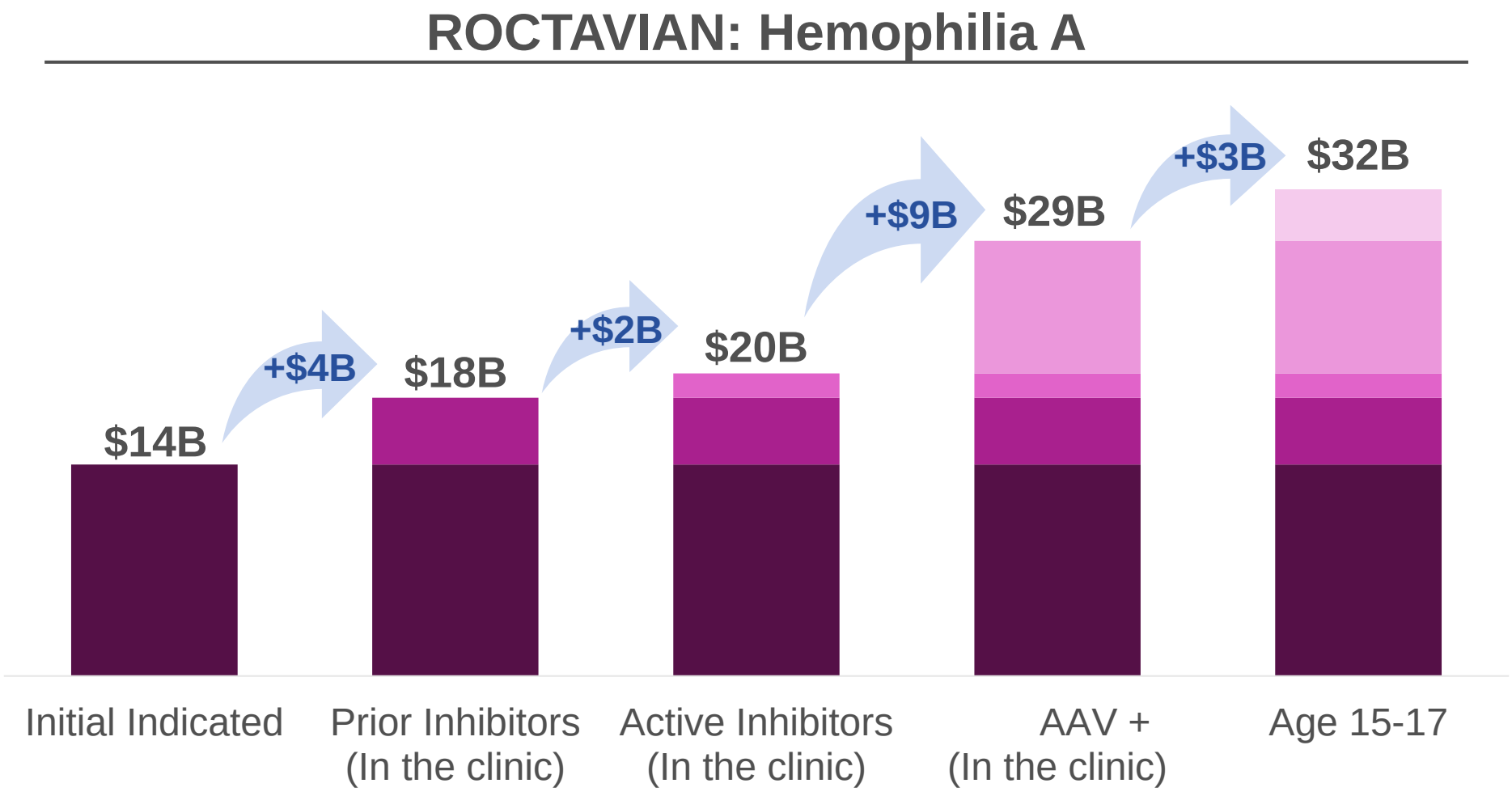
GSS conditions currently in the clinic:

- 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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Multi-Billion Dollar Cumulative Potential Addressable Market Opportunity[1] Beyond ROCTAVIAN Initial Indication



[1] ROCTAVIAN market represents a one-time treatment per patient and not annual re-occurring opportunity

Leveraging Proven Manufacturing Capabilities to Further De-risk Next Clinical Assets

Wholly Owned Manufacturing Capabilities Support Rapid Drug Development and Commercialization

Gene Therapy Facilities



- **2017:** Novato, California facility commissioned (ISPE 2018 Facility of the Year – Project Execution)
- **Footprint:** 50,000ft^2 @2000L Bioreactor Scale
- **Manufactures**: ROCTAVIAN Drug Substance + Sterile DP

- **2020:** New Gene Therapy Pilot Plant commissioned
- **Footprint:** 6,500 ft^2 @500L and 2000L Bioreactor Scale
- **Manufactures:** New Gene Therapy Clinical Candidates

Biologic Facilities



- **1999:** Novato, California facility opened
- **Footprint:** 80,000ft^2
- **Manufactures**: ALDURAZYME, NAGLAZYME, PALYNZIQ, VIMIZIM, VOXZOGO Drug Substance



- **2011:** Shanbally, Ireland facility acquired
- **Footprint:** 200,000ft^2
- **Manufactures:** BRINEURA, VIMIZIM

- **2023**: Sterile Drug Product Capability

Substantial Track Record of Global Inspections and cGMP Compliance

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Our Strategic Priorities To Drive Sustainable Growth & Value Creation



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ROCTAVIAN PHASE 3 3-YEAR and 4-Year RESULTS

		Phase 3 6e13 vg/kg dose	
		In Year 3 N=132/112*	In Year 4 N=17**
FVIII Activity	Mean	18.8	15.2
	Median	8.4	7.4
Annualized Bleeding Rate*** (bleeding episodes per year)	Mean	1.0	0.8
	Median	0.0	0.0
Annualized FVIII Utilization (infusions per year)	Mean	8.4	11.1
	Median	0.0	0.0

**N=132 (FVIII Activity); N=112 (ABR and AFR). Two of these patients discontinued from the study prior to reaching Year 3. FVIII imputed to be 0 IU/dL; no imputation was carried out for ABR and AFR.*

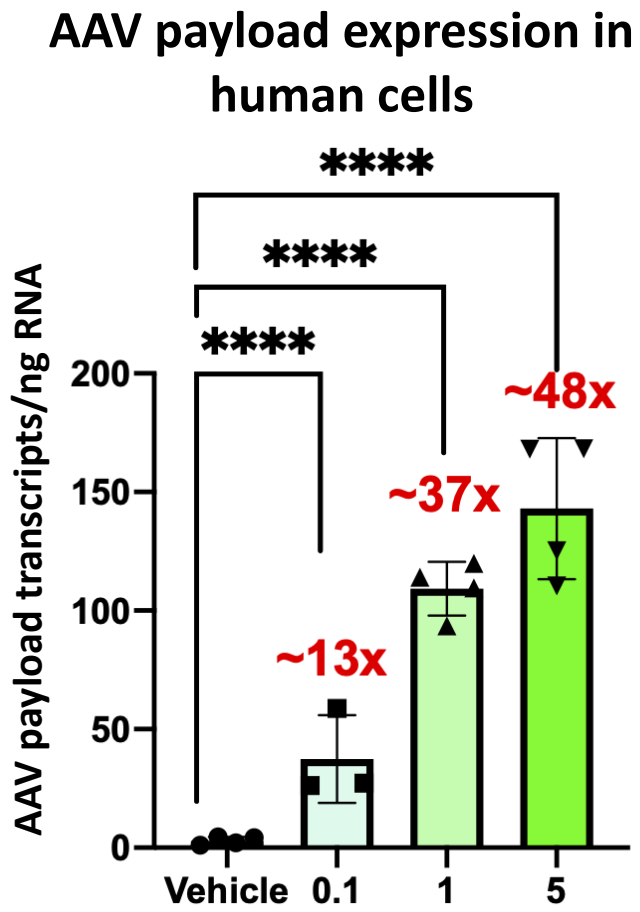
***N=17. One of these patients discontinued from the study prior to reaching Year 4. FVIII activity imputed to be 0 IU/dL; no imputation was carried out for ABR and AFR.*

****Annualized rate is for treated bleeds.*

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Identification of Mechanisms Regulating AAV Episomes Enables Increased Expression with an Approved Small Molecule Drug

- Non-clinical and clinical studies of AAV episomes indicates that transgene expression is restricted by a transcriptional mechanism
- Screen of approved small molecule drugs modulating this mechanism identified several promising candidates that increase transgene expression
- Next steps: Investigate in humans the potential of these compounds to augment initial expression, to extend or enhance sustained expression and reignite expression if lost



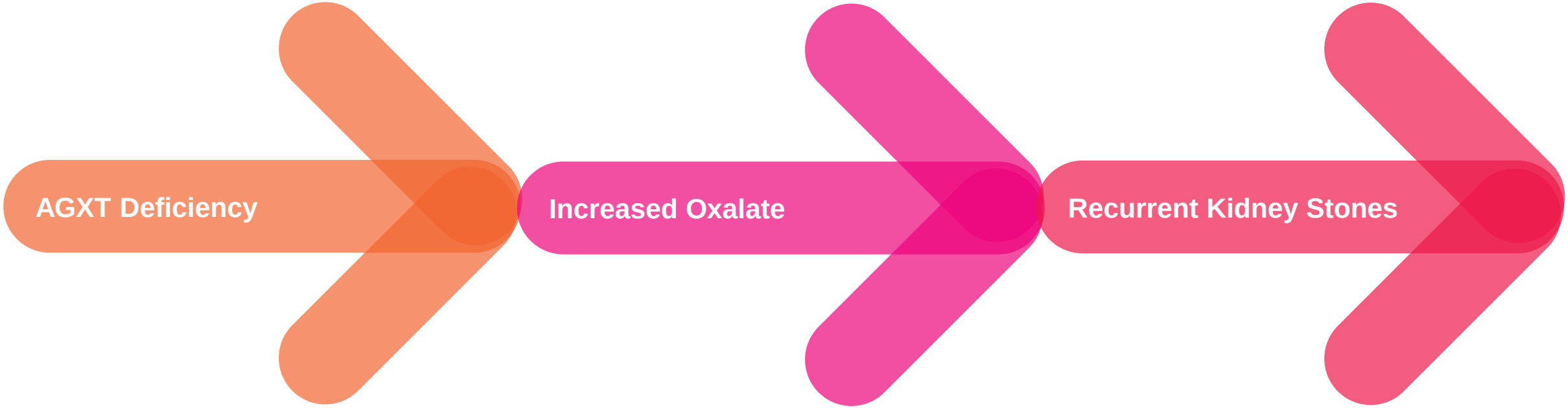
Concentration of approved drug tested relative to Cmax of approved dose

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

	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+***		

Inspiration for BMN 255: Progressive Renal Failure and Recurrent Kidney Stones in Primary Hyperoxaluria Type 1

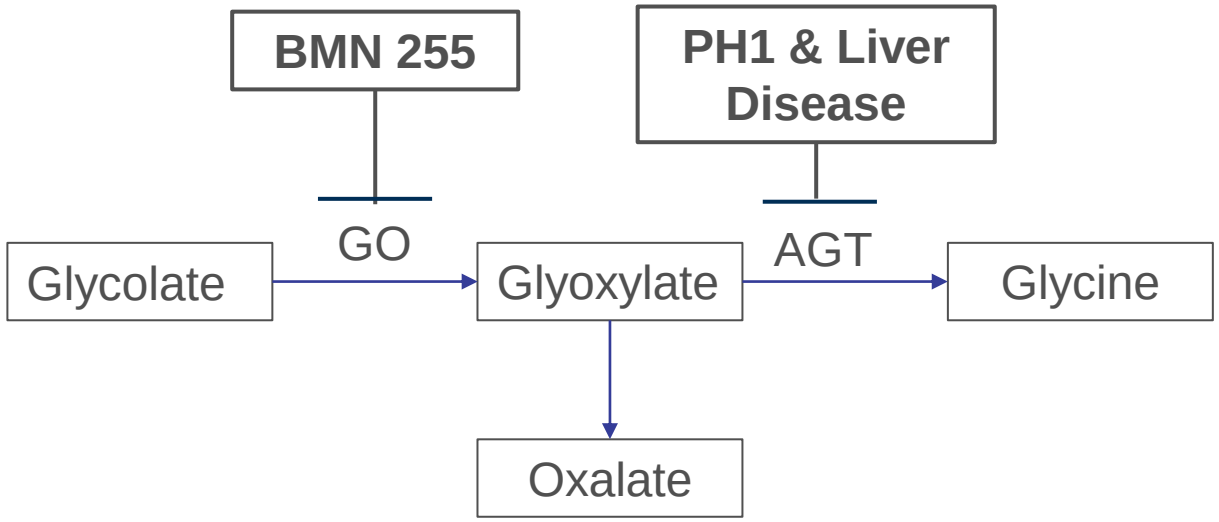


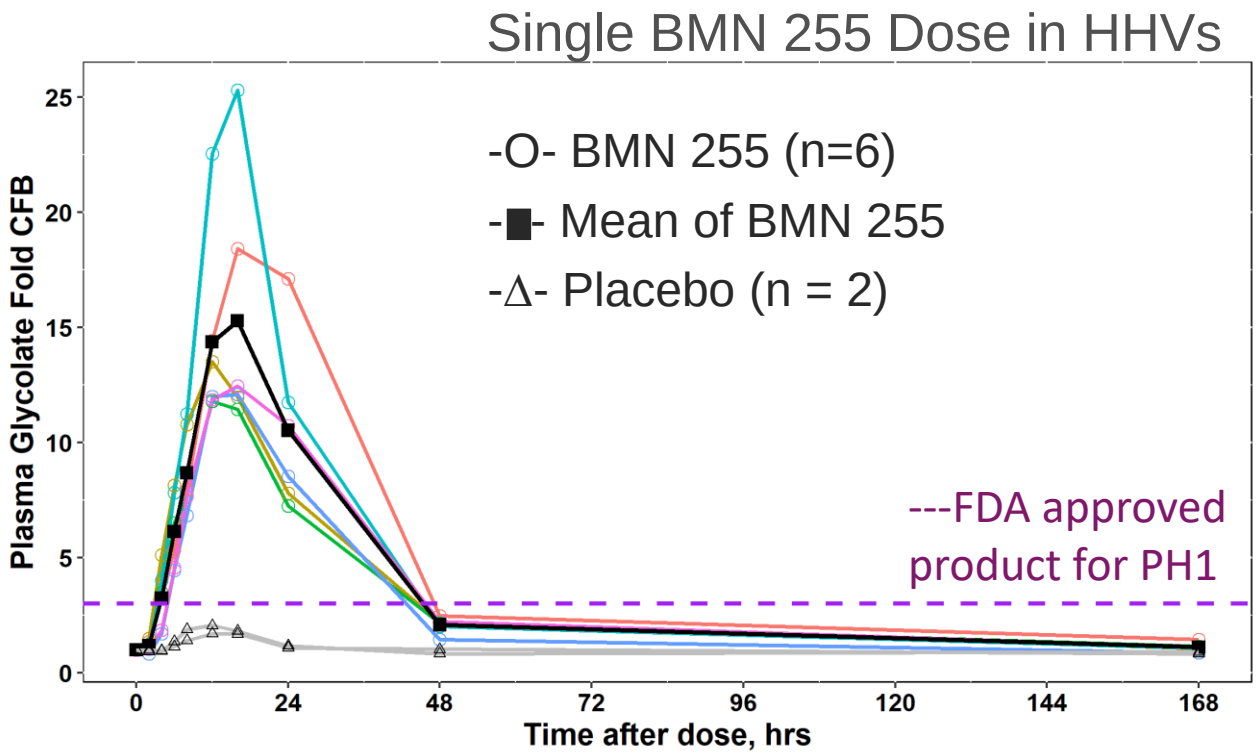
In PH1 Inherited mutations disrupt the AGXT gene → Reduced AGXT function causes increased oxalate → Oxalate crystals lead to recurrent kidney stones and kidney damage

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BMN 255 is a Small Molecule Glycolate Oxidase (GO) Inhibitor

Multi-ascending dose (MAD) Phase 1/2 underway





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BMN 331 for Hereditary Angioedema (HAE): HAE Imposes a Life-Long Risk of Attacks

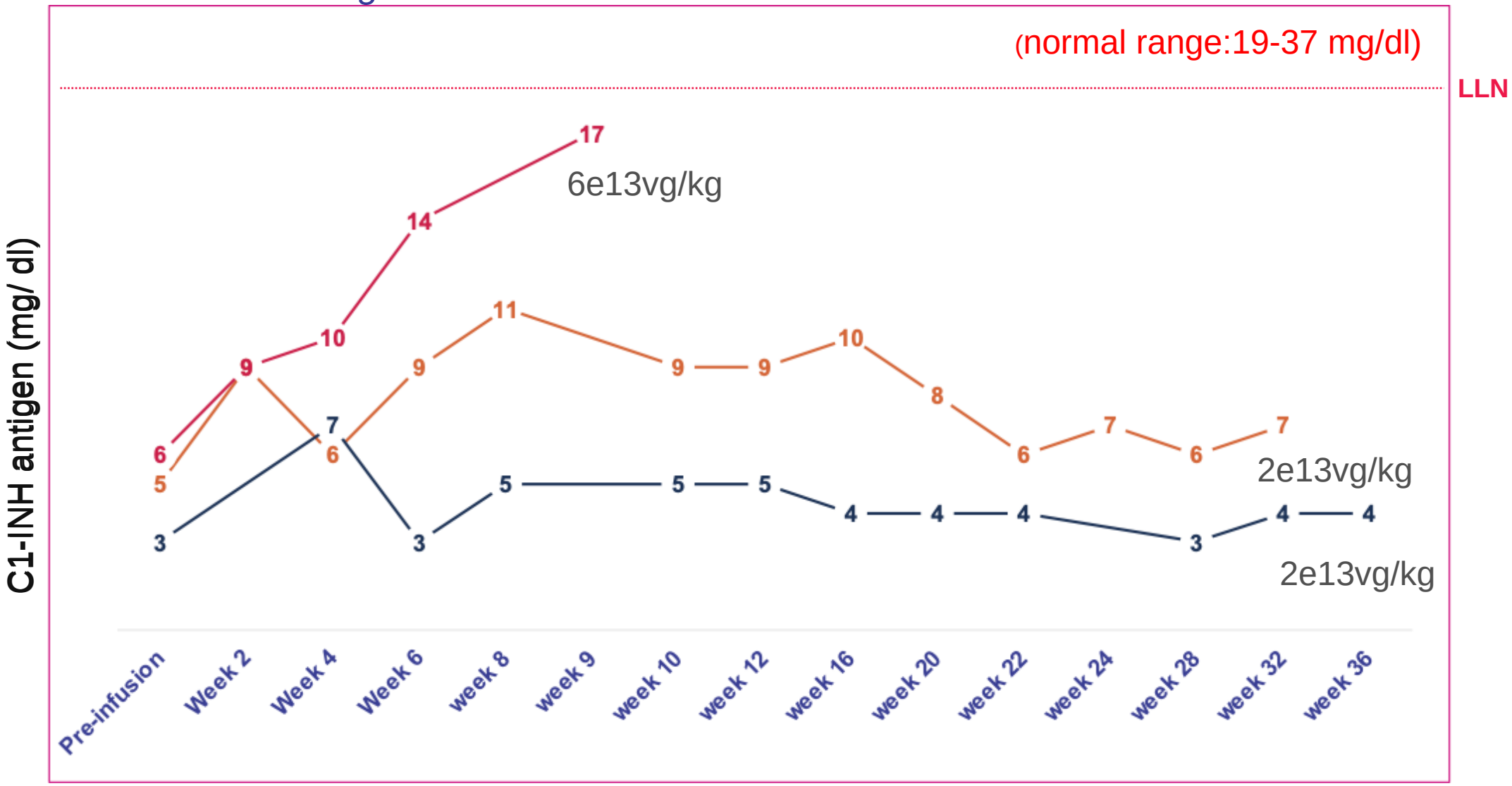


- Available therapies for HAE cannot provide enduring compensation for loss of C1-inhibitor activity
- “Troughs” of activity or missed treatment can leave individuals with HAE exposed to severe attacks
- Pre-clinical studies with BMN331 in *SerpinG1* mutant mice and NHPs suggest that a 6 E13 dose of BMN331 may provide ample expression of C1-INH within an acceptable range of variation to durably protect recipients from HAE attacks

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BMN 331 AAV Gene Therapy for Hereditary Angioedema (HAE)

331-201: First 6 E13 dosing occurred in October 2022



C1-INH antigen levels (mg/dL) by visit

Anticipated 2023 INDs Offer Multiple Best-in-class Product Candidates

Therapeutic Focus	Product Candidate / Key Attributes
Musculoskeletal/ Metabolic	**BMN 351 DMD** (Exon 51 Oligonucleotide) • > 10x fold enhanced potency by targeting novel splice enhancer • At anticipated muscle concentrations > 20% dystrophin predicted
	BMN 349 A1ATD (Small Molecule) • Rapidly acting, orally bioavailable, binds to mutant A1AT • Reduces polymer burden and improves liver health in mouse model
Cardiovascular	**BMN 293 MYBPC3 HCM** (Gene Therapy) • Cardiac specific C3 gene addition delivered by AAV • Durable rescue of hypertrophy and function in animal disease model

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Building on Our Strategic Pivot to Larger Patient Populations with Serious Unmet Medical Needs

Therapeutic Focus	Product Candidate	Addressable Patient Population [1]
Musculoskeletal / Metabolic	**BMN 351 DMD** (Exon 51 Oligonucleotide)	~8K
	BMN 255 Hyperoxaluria (Small Molecule)	~800K
	BMN 349 A1ATD (Small Molecule)	~52K
Cardiovascular	**BMN 331 HAE** (Gene Therapy)	~13K
	BMN 293 HCM (Gene Therapy)	~180K

[1] Clinically addressable patient population across BioMarin territories

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



BIOMARIN®

Phenylketonuria

KUVAN® (sapropterin dihydrochloride)

Palynziq™ (pegvaliase) Injection

Hemophilia A Gene Therapy

ROCTAVIAN® (valoctocogene roxaparvovec) Solution for intravenous infusion

Our Strategic Priorities To Drive Sustainable Growth & Value Creation



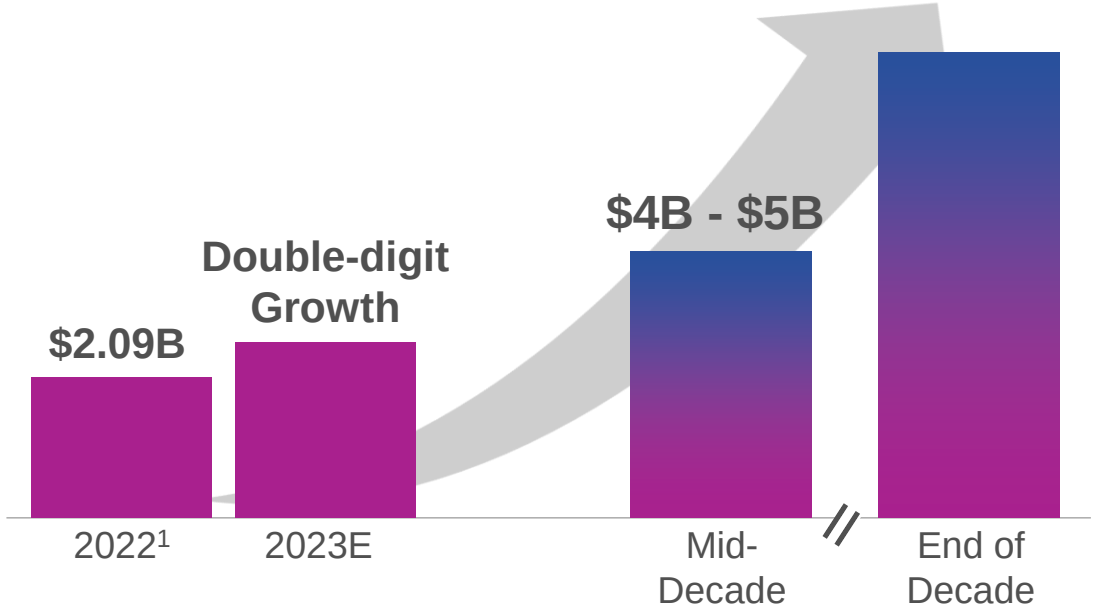
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Accelerating Revenue and Profitability Through Innovation

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

[1] Unaudited preliminary 2022 revenue estimate

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Evolving Financial Reporting Metrics Beginning in 2023

GAAP to Non-GAAP Reconciliation

GAAP Net Income	
Interest income (expense), net	
Provision for (benefit from) income taxes[1]	
Depreciation expenses	
Amortization expense[2]	●
Stock-based compensation expense	●
Significant non-recurring items	●
Non-GAAP Net Income	

P&L Guidance Measures

P&L Line	GAAP	Non-GAAP
Total Revenue	●	●
Gross Profit *% of Revenue*	●	
R&D *% of Revenue*	●	
SG&A *% of Revenue*	●	
Net Income (Loss)	●	●
Diluted EPS	●	●

[1] We will continue to adjust for tax effects of individual non-GAAP adjustments but not the entirety of income taxes [2] To include amortization of intangible assets only

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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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Thank You!