BIOMARIN

36th Annual J.P. Morgan Healthcare Conference

Jean-Jacques Bienaimé
Chairman and Chief Executive Officer
BioMarin Pharmaceutical Inc.

2018

Safe Harbor Statement

This non-confidential presentation contains 'forward-looking statements' about the business prospects of BioMarin Pharmaceutical Inc., including potential future products in different areas of therapeutic research and development. Results may differ materially depending on the progress of BioMarin's product programs, actions of regulatory authorities, availability of capital, future actions in the pharmaceutical market and developments by competitors, and those factors detailed in BioMarin's filings with the Securities and Exchange Commission such as 10-Q, 10-K and 8-K reports.

BIOMARIN

2017 Key Accomplishments

Clinical	Presented positive data with Valoctocogene Roxaparvovec	1Q17
	Presented positive data with BMN 250	3Q17
	Dosed first patient in Phase 3 study with Valoctocogene Roxaparvovec	4Q17
Regulatory	Received PRIME designation for Valoctocogene Roxaparvovec in EU	1Q17
	Received approval from FDA and EMA for Brineura	2Q17
	Filed BLA for Pegvaliase	2Q17
	Received Priority Review for Pegvaliase	3Q17
	Received Breakthrough Designation in the US for Valoctocogene Roxaparvovec	4Q17
Financial	Sold PRV for $125 million	4Q17
	Non-GAAP Profitable (Guidance)	FY17E

BIOMARIN

6 Commercialized Products and 5 in Development

Commercialized Products

Brineura® (cerliponase alfa)

Naglazyme® (GALSULFASE)

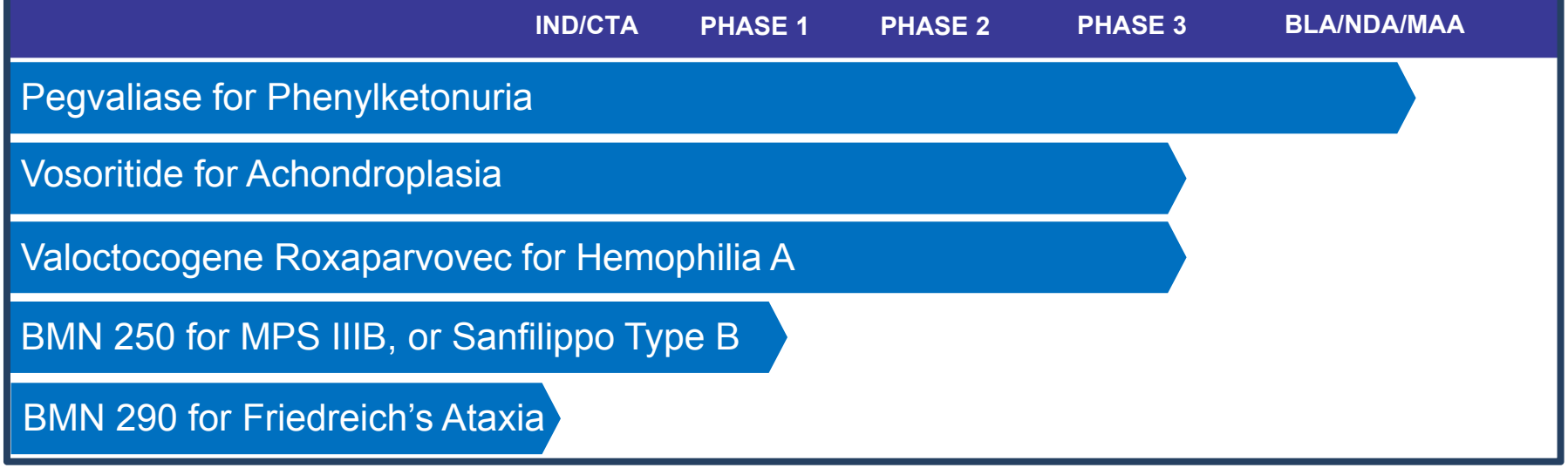
KUVAN® (sapropterin dihydrochloride) Tablets

ALDURAZYME® (LARONIDASE)

VIMIZIM® (elosulfase alfa)

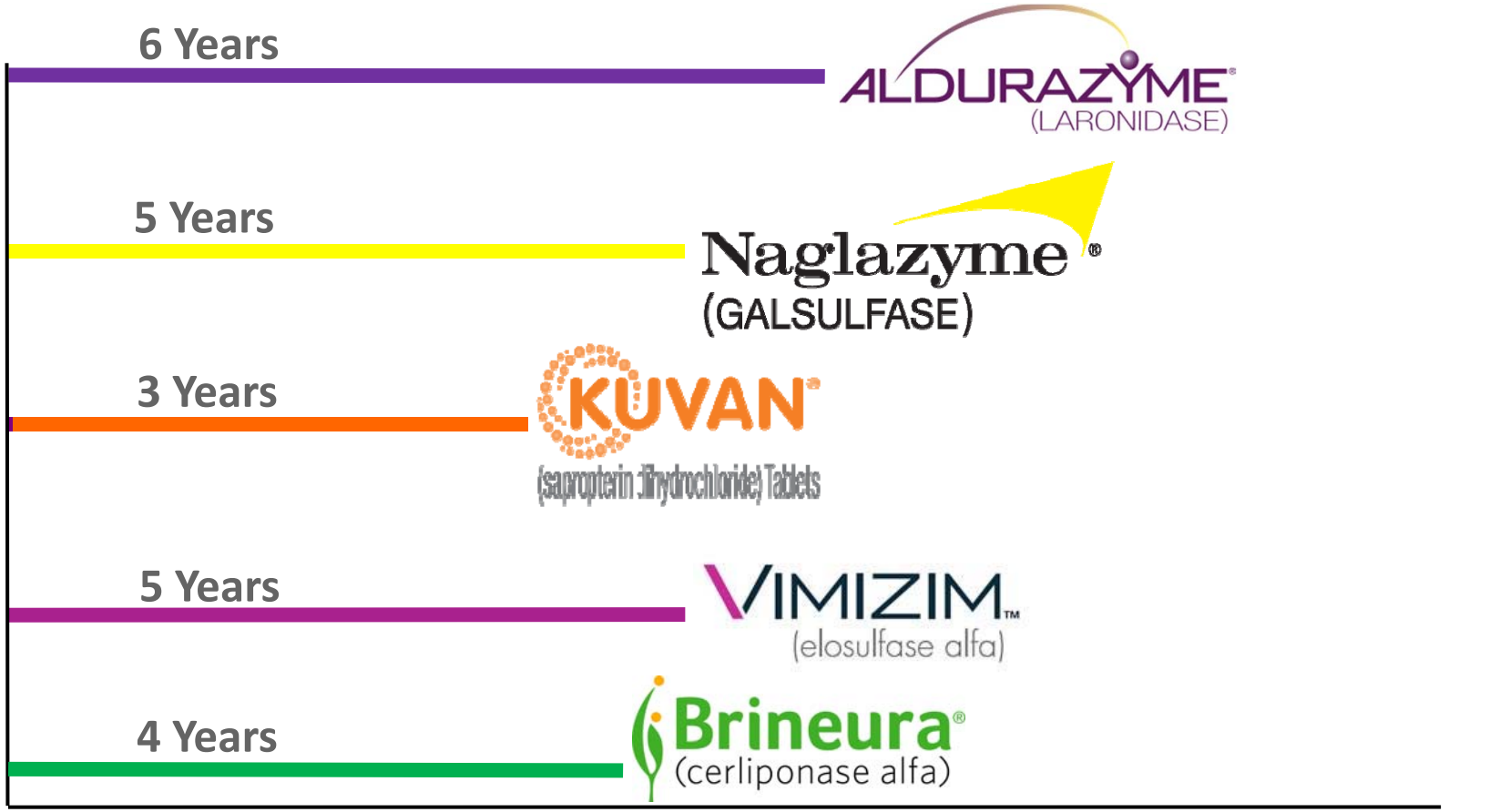
FIRDAPSE™ amifampridine

Product Development Pipeline



BIOMARIN

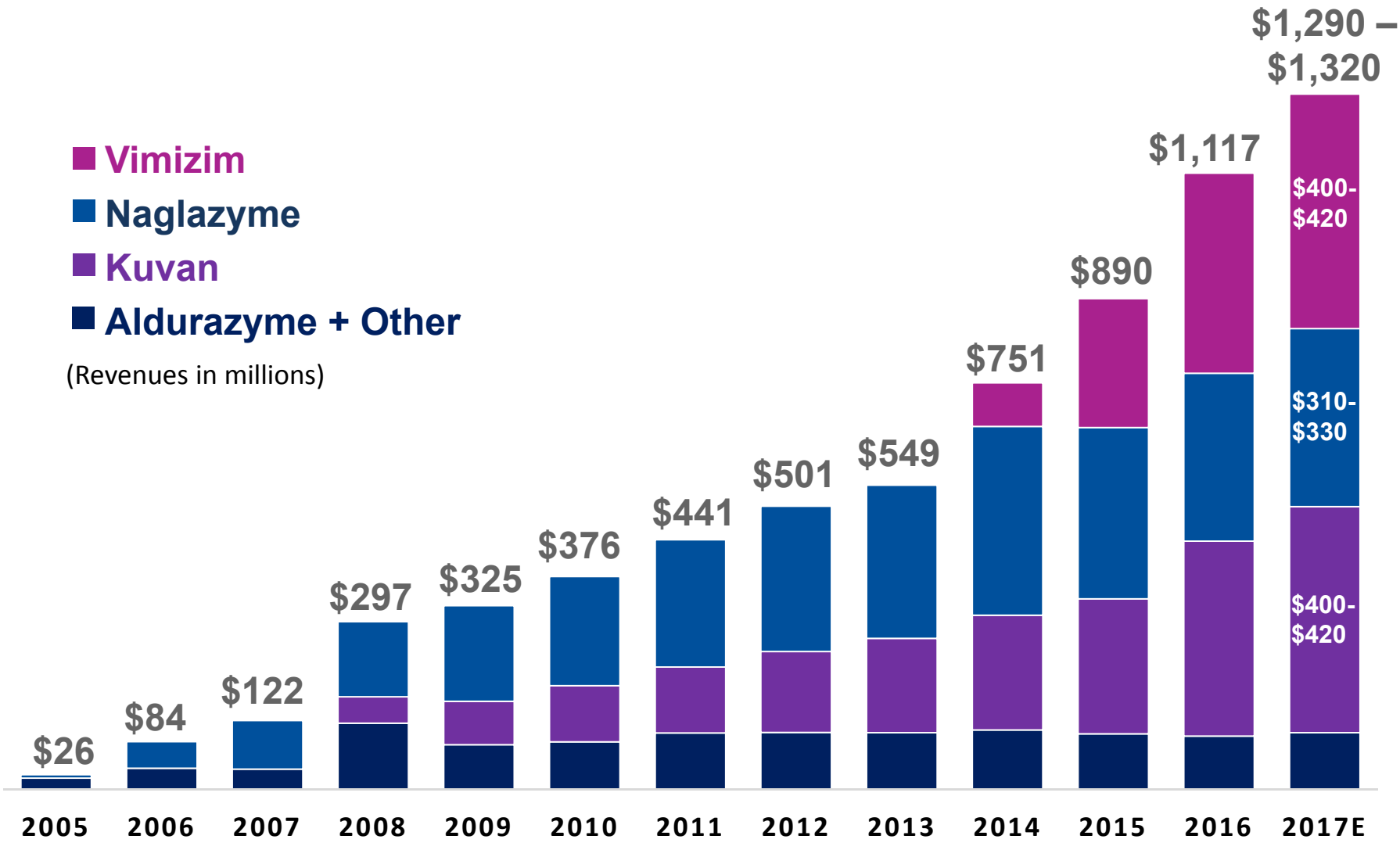
Efficient Drug Development Drives Strong Returns on R&D Investment



Time to Approval from IND/CTA filing

BIOMARIN

Demonstrated Track Record of Consistent Revenue Growth



BIOMARIN

Our 7th potential commercial opportunity:

Pegvaliase for Phenylketonuria (PKU)

Target population:
Adult patients with PKU who have uncontrolled blood phenylalanine (Phe) concentrations

Kuvan and Pegvaliase: Complementary Therapies for PKU

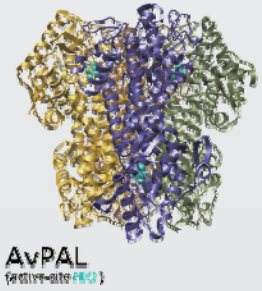
PKU: Inability to break down Phe leads to toxic levels in the brain

Kuvan® - approved in 2007
(sapropterin dihydrochloride)

- Targets residual phenylalanine hydroxylase (PAH)/modestly affected patients
- Modestly effective, requires severe restriction of protein intake
- Mostly used in children

Pegvaliase – PDUFA May 2018
(phenylalanine ammonia lyase)

- Substitutes for missing enzyme/ appropriate for even most severe
- Substantial Phe lowering even in the face of near normal protein intake
- Very useful for adults who want to have a more balanced nutritional intake

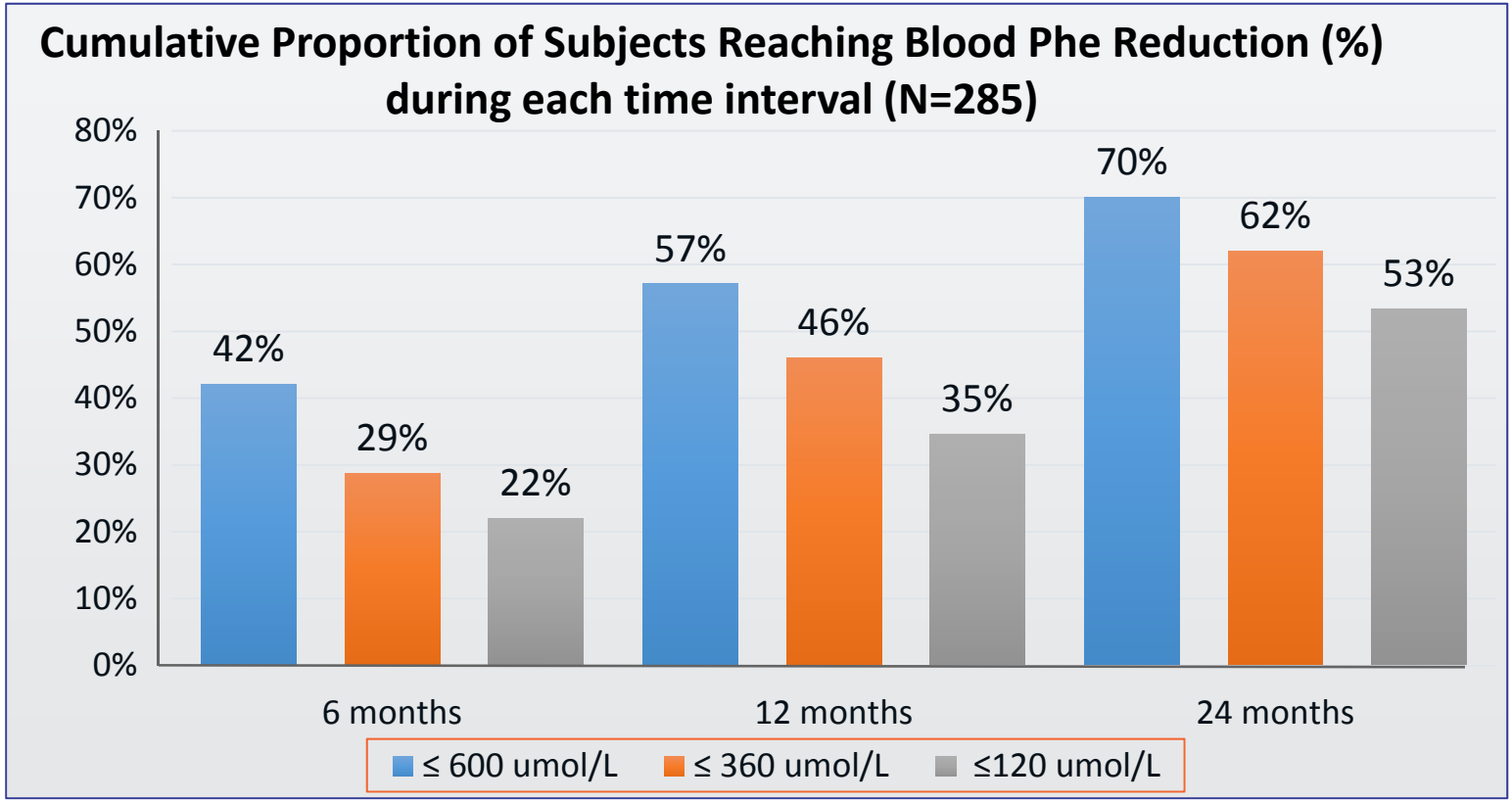


In Phase 3 Study, Majority of Subjects Achieved Blood Phe Reduction to Treatment Guidelines

≤ 360 µmol/L - US guideline recommendation

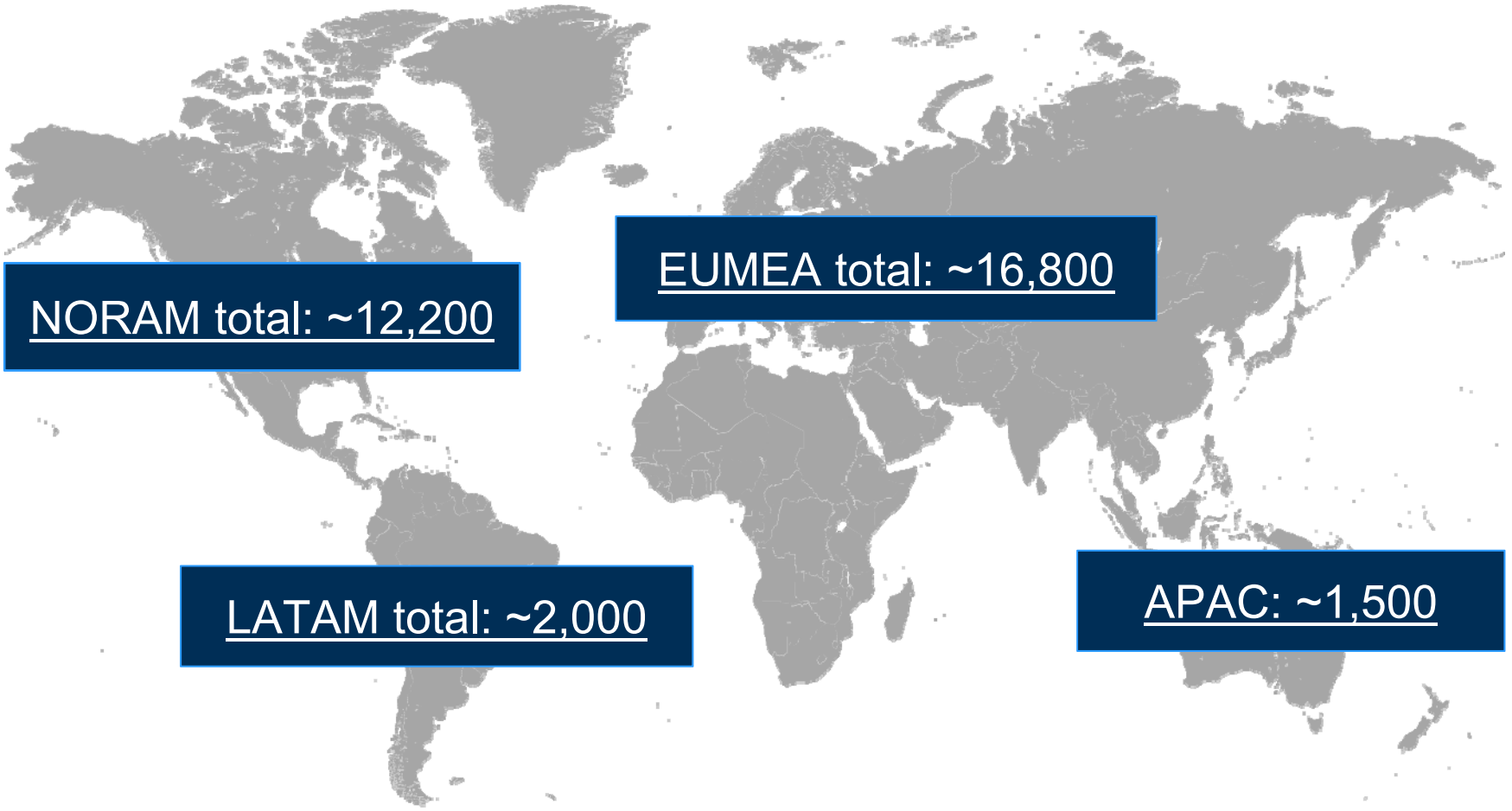
≤ 600 µmol/L - EU PKU guideline recommendation

≤ 120 µmol/L - physiologically normal



Subjects reflect general adult PKU population with mean baseline blood Phe 1233µmol/L

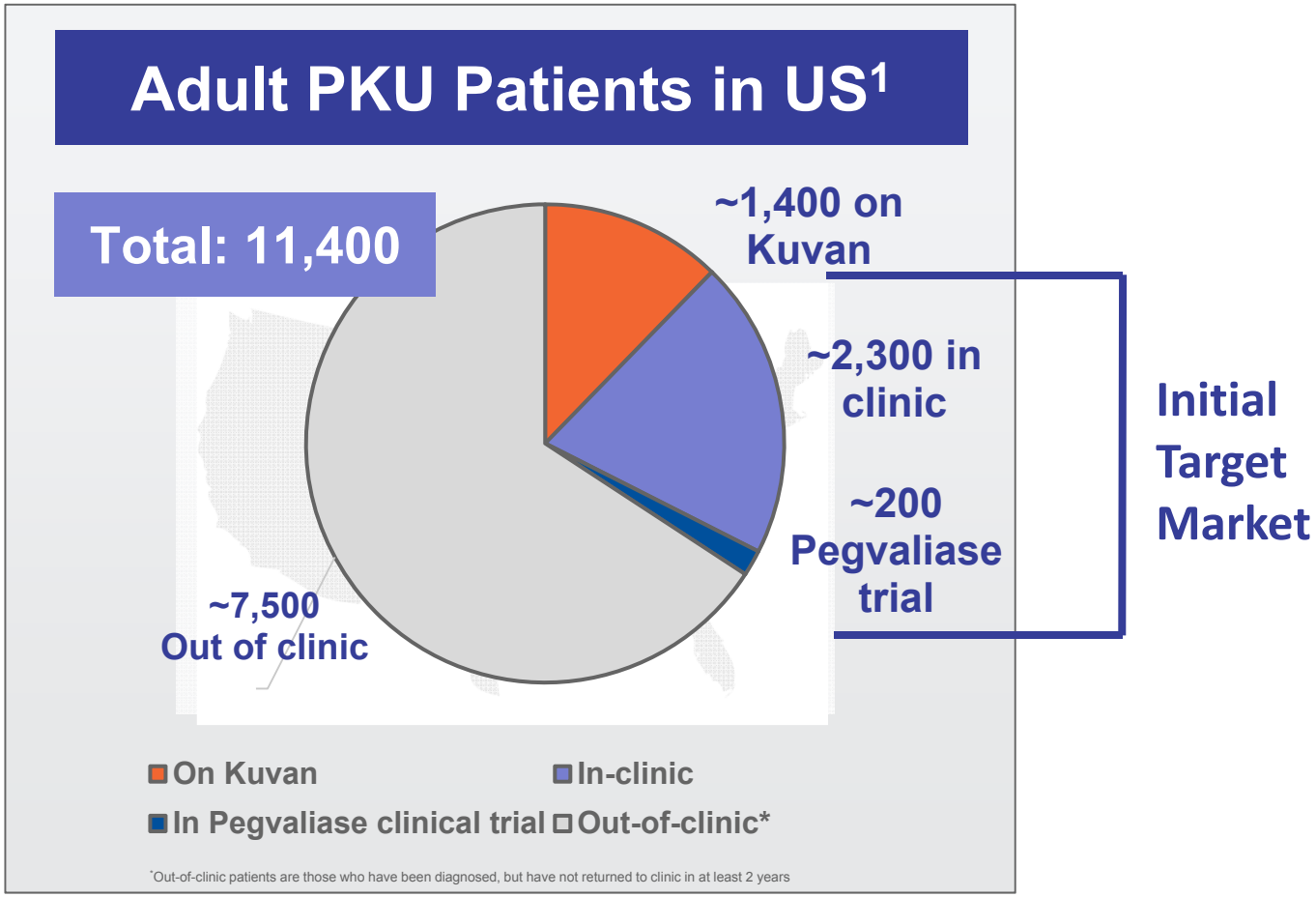
An Estimated 33K PKU Adults in our Territories[1]



[1]*Population based on 53 Kuvan markets (excluding China); estimates using country-level PKU incidence, start of NBS program and coverage of live births over time*

PKU Patients are Diagnosed at Birth

10 years of commercial experience with Kuvan will facilitate Pegvaliase launch; Annual net cost for an adult Kuvan patient is $150K per year

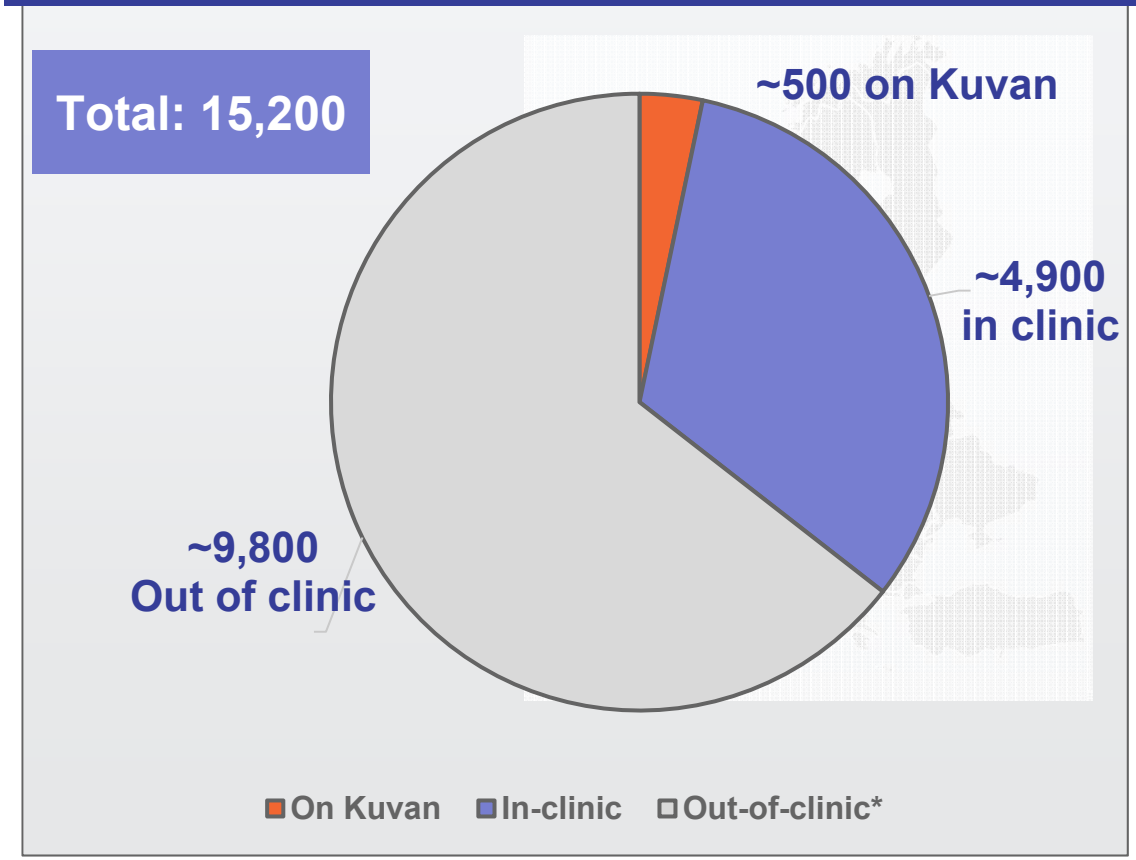


[1]PKU patients defined as patients diagnosed through newborn screening
Note(1): US, EU, and Turkey represents up to 90% of total adult market in BMRN markets
Note(2): total in-clinic PKU population (pediatric and adult) in BMRN markets estimated to be ~50,000 patients

Significant Markets in EU and Turkey

2016 guidelines in EU for PKU management builds foundation for Pegvaliase

Adult PKU Patients in EU and Turkey[1]



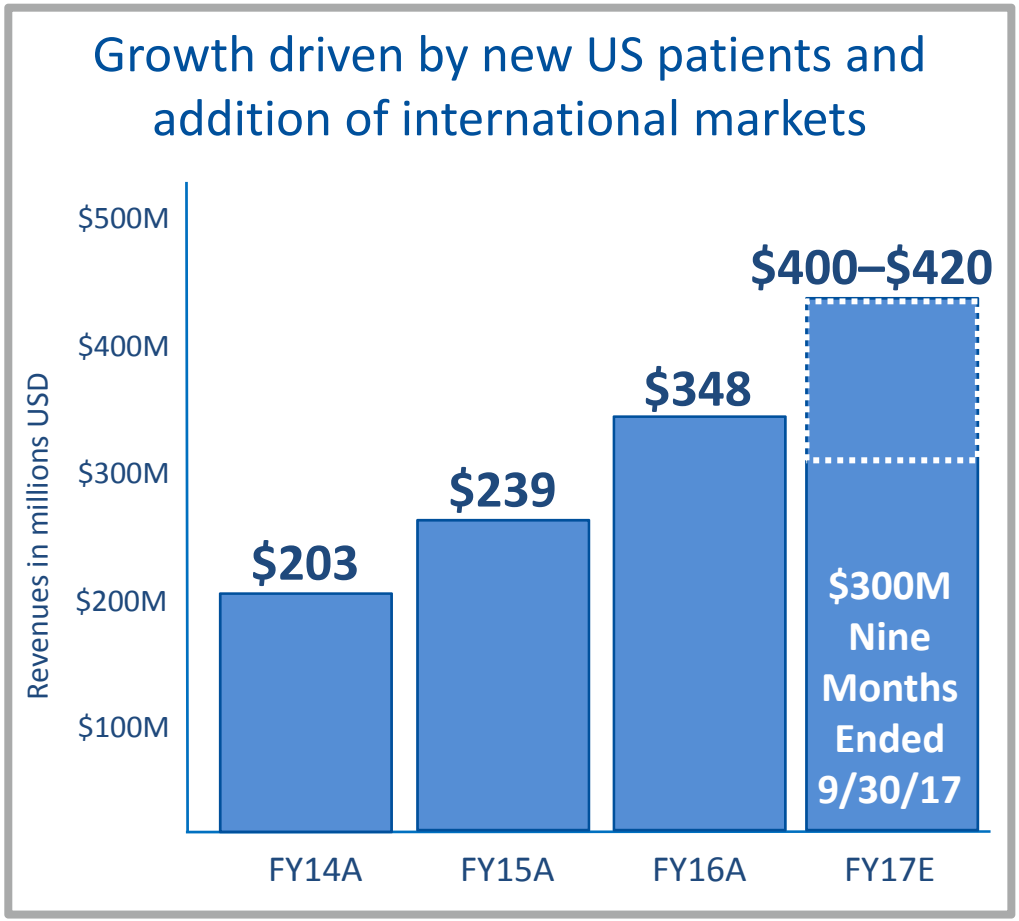
[1]PKU patients defined as patients diagnosed through newborn screening
Note(1): US, EU, and Turkey represents up to 90% of total adult market in BMRN markets
Note(2): total in-clinic PKU population (pediatric and adult) in BMRN markets estimated to be ~50,000 patients

Kuvan FY 2017 revenue guidance $400M-$420M

16% Growth First Nine Months of 2017

Significant Worldwide Opportunity

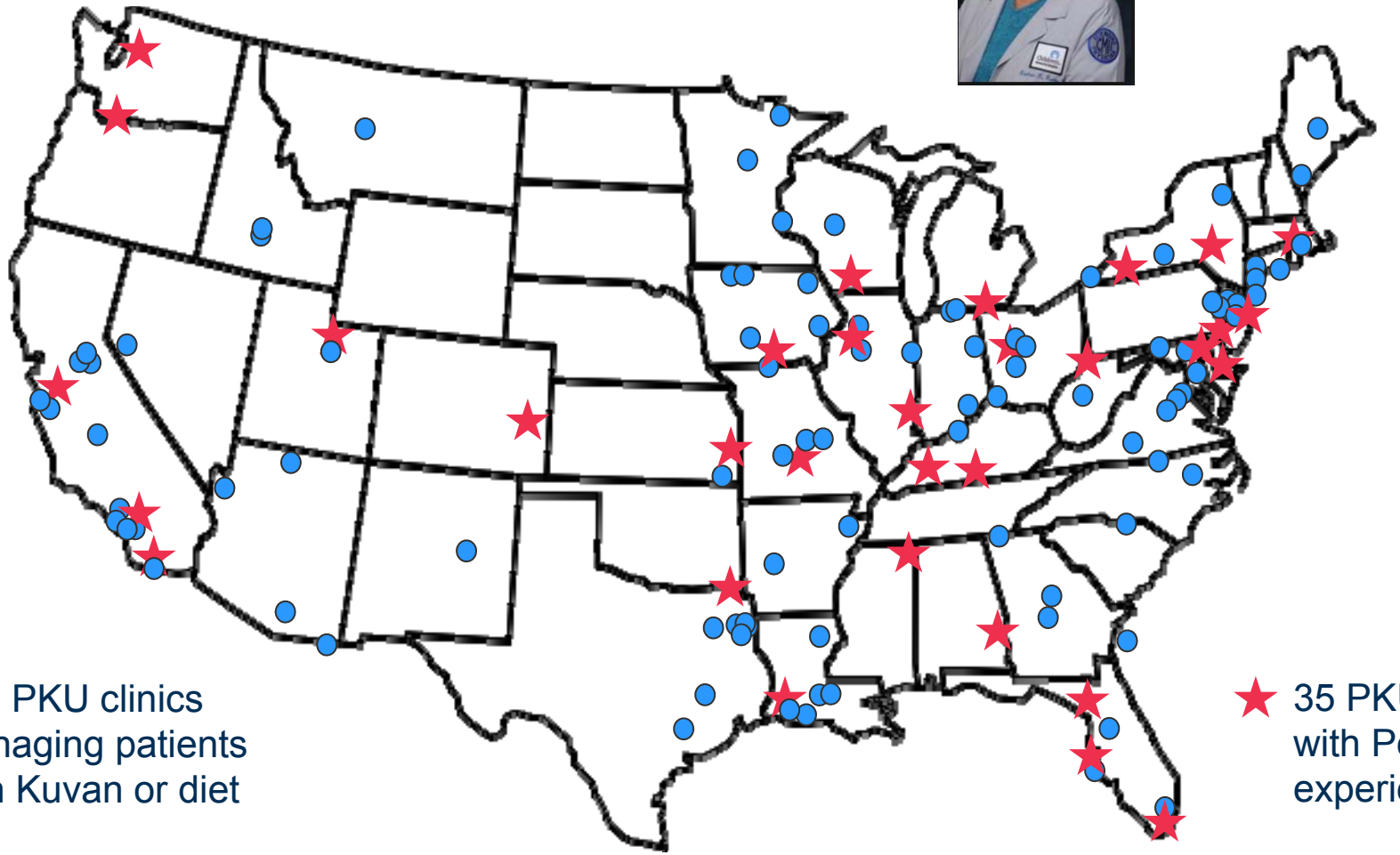
- EU exclusivity extended through 2024
- Patient growth 15% Y/Y in NorAm region
- Developing market to prepare for potential pegvaliase launch



Broad Network of Experience in PKU in Advance of Pegvaliase Launch

"From the perspective of a clinician who has treated PKU day in and day out my entire career, this Phe reduction is absolutely mind blowing"

Dr. Barbara Burton, Lurie Children's Hospital, Northwestern University



- 125 PKU clinics managing patients with Kuvan or diet
- 35 PKU clinics with Pegvaliase experience

Successful Kuvan Commercialization Provides Base for Pegvaliase Launch

Kuvan and Pegvaliase offer a portfolio of products for the PKU community

- ✓Field based clinical support for Pegvaliase induction and titration schedule
- ✓Robust educational materials for HCPs and patients
- ✓Seamless access and distribution of Pegvaliase across multiple doses and injection schedules throughout the induction and titration period

BIOMARIN Rare Connections™

BIOMARIN Clinical Coordinators

Pegvaliase: Path to Potential Approval 1H18

2H18 US pegvaliase launch if approved at May PDUFA

Status

- BLA file accepted
- Priority Review granted
- Advisory Committee meeting not anticipated

Next Steps

- Continued market preparations for US launch
- May 25, 2018 PDUFA
- Expect MAA submission 1Q18

BIOMARIN

Vosoritide

BMN 111

Target indication:

Achondroplasia

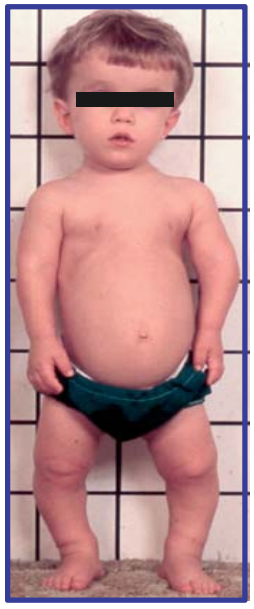
Achondroplasia: Most Common form of Dwarfism

Spontaneous mutation that occurs in 80% of cases from parents of average stature

In addition to short stature, serious medical complications include:

- foramen magnum compression
- sleep apnea
- bowed legs
- permanent sway of the lower back
- spinal stenosis
- obesity

Children with Achondroplasia Grow an Average of 4cm/year vs. 6cm/year for Average Height Children



Market Opportunity is Significant with Vosoritide

80% of patients are in BioMarin's global territories ex-US

	North America	EUMEA	Latin America	Asia Pacific	*Patients in Global BMRN Territories*
People with achondroplasia: all ages	14,200	43,700	25,000	12,600	95,500*
Addressable market: children with open growth plates	3,500	10,900	6,250	3,200	**~24,000**

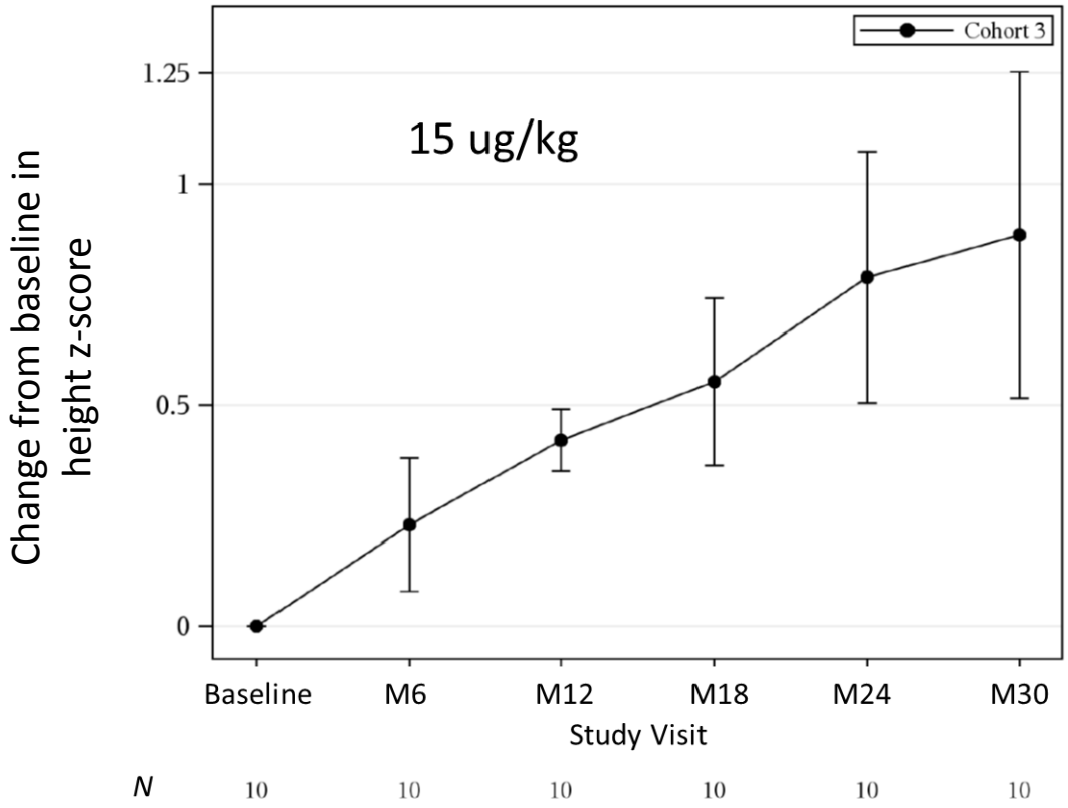
Source: the CIA World FactBook 2014 edition

- 1:25,000 live births
- No regional variance

Durable Growth Effects in Children with Achondroplasia Treated with 15 ug/kg Vosoritide for 30 months

Results to date suggests treatment provides additional 4cm of growth at 30 months

Height Z-score increased close to 1 Standard Deviation score after 30 months



*Whisker bars represent one standard deviation above and below mean

Enthusiasm for Vosoritide Driving Brisk Demand for the Phase 3 Study

Great anticipation that vosoritide could positively impact disease burden and QoL

"Achon parents want to avoid complications, orthopedic issues, issues with mobility, better stamina/exercise tolerance" – Pediatrician (Canada)

"It's not just about lengthening bone. Avoiding surgery is key" – Orthopedic Surgeon (UK)

"50% increase in growth velocity would be a miracle…it's almost too good to be true" – Pediatric Neurologist (Canada)

Source: BioMarin Market Research

BIOMARIN

Vosoritide Development Program Goal to Demonstrate the Ability to Improve Clinical Outcomes

4 pronged program in children with achondroplasia to support global approval

Phase 3 study, global, placebo-controlled for unequivocal proof of efficacy
- N=125
- Annualized Growth Velocity (AGV) improvement for a minimum of 1 year
  - 2-3 years or more data by potential PDUFA

Long-term, open-label Phase 2 program to corroborate maintenance of effect
- N=23
- Over 5 years of clinical data anticipated at filing
- 23 patients will reach 85% of final adult height by anticipated filing in 2020

Infant/toddler study in 1H18 to validate safety of starting treatment early
- N=60; PIP in place in EU
- Goal to safely dose as early as possible for maximal effect
- Endpoints include safety/tolerability, growth, QoL, PK and biomarkers

Natural History program to augment clinical understanding of outcome
- Longitudinal and relationship of stature to functional impairment studies of height velocity/height through adulthood of untreated patients for comparison to treated patients
- Cross sectional studies to investigate statural and functional impairment
- Assess minimally important clinical difference in final height gain

Endpoints in Achondroplasia

Challenges: achondroplasia is heterogenous

- Not all patients have all manifestations
- Not all manifestations are completely reversible
- Rare condition so must study the prevalent population

Opportunities to leverage stature gain as primary endpoint

- Most sensitive endpoint
- Strong regulatory history and acknowledgment of meaningfulness of final adult height in achondroplasia to support approvals
- Can build bridge from height velocity to final stature through combination of long-term extension studies and natural history studies of untreated patients
- Can augment picture of improved overall health through long-term studies (generally not feasible in shorter-duration studies)

Collaborating with FDA to establish guidance for drugs in development

- As a leader in the field of rare disease drug development, we welcome opportunity to establish a high bar

BIOMARIN

Valoctocogene Roxaparvovec
(BMN 270)

Target indication:

Severe Hemophilia A

Rationale for Valoctocogene Roxaparvovec Program

Potentially eliminates the need for chronic treatment in severe hemophilia A

Limitations of Current Standard of Care

High Unmet Medical Need

- Breakthrough bleeds common
- Factor augmentation needed for less severe incidents, e.g., falling down
- Quality of Life impacted by constant need for factor replacement

Gene Therapy Benefits

One Time Treatment Option

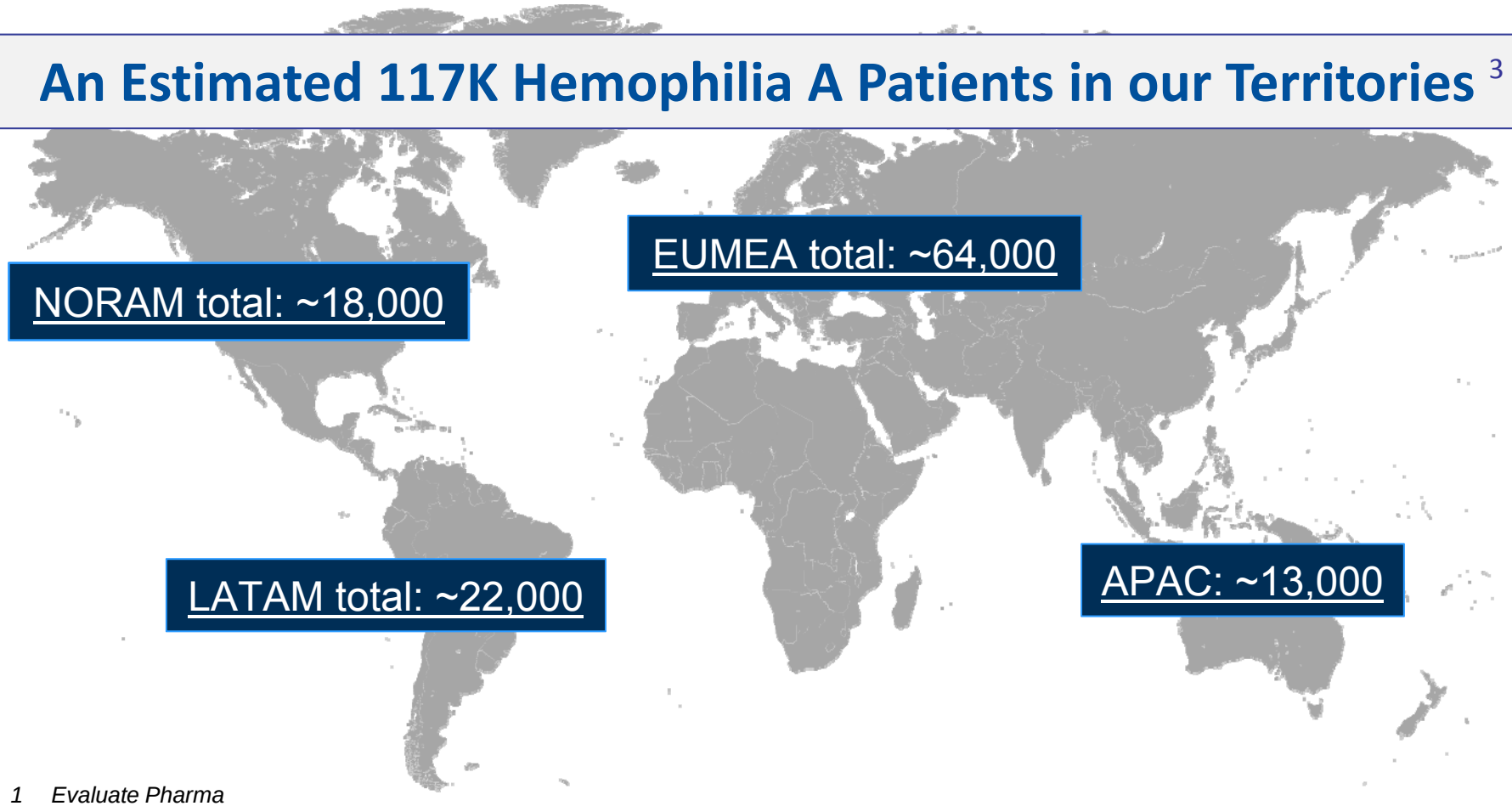
- Maintains constant expression at normal levels
- Eliminates need for supplemental infusions
- Restores hemostasis and eliminates bleeds

BIOMARIN

Global Hemophilia A Market in 2016 was $8.4B[1]

Fully-compliant, WAC pricing for FVIII replacement is $403K-$674K per year [2]

An Estimated 117K Hemophilia A Patients in our Territories [3]



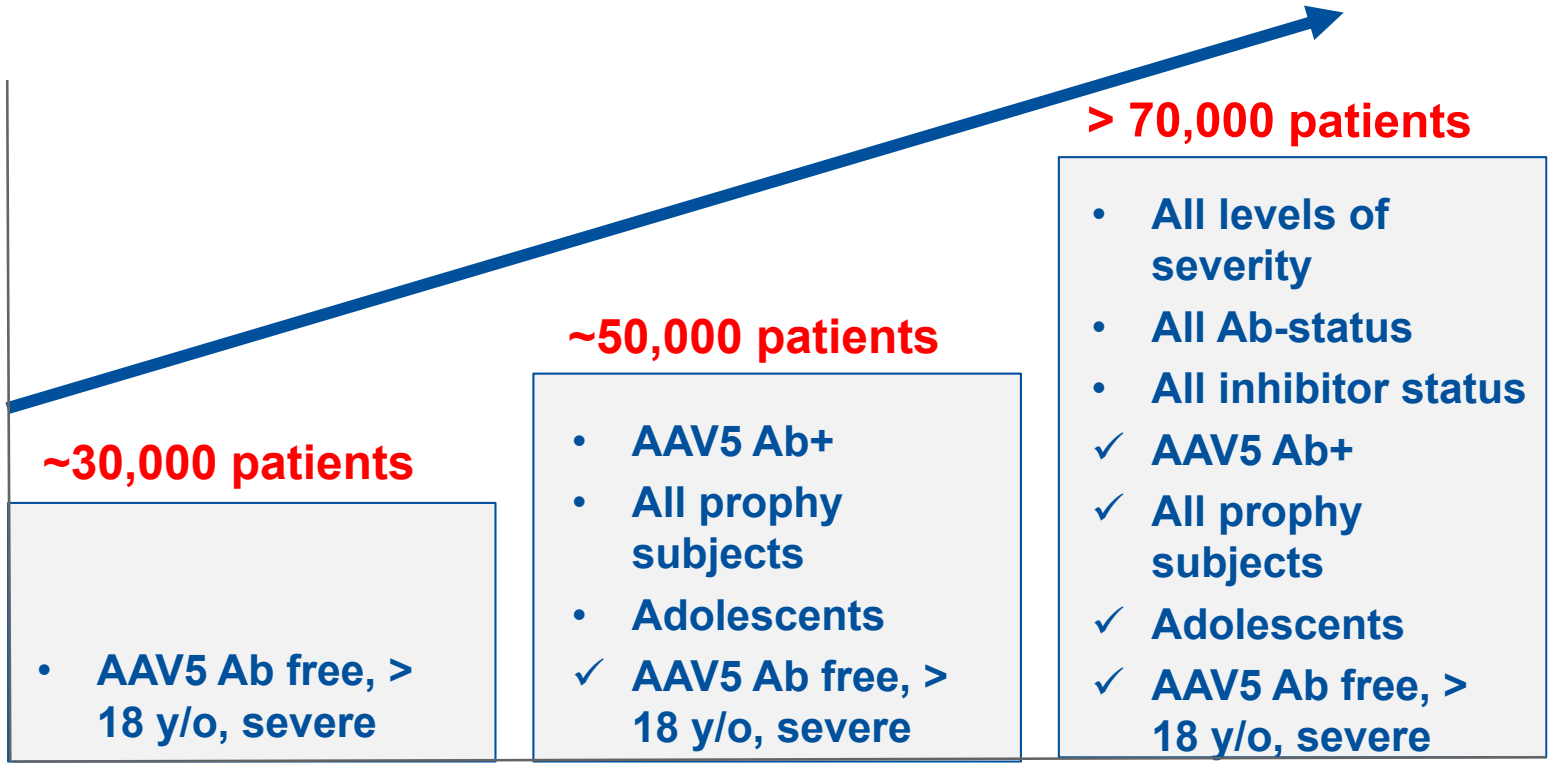
1 *Evaluate Pharma*

2 PriceRx IHA Global insight Oct. 2015-Oct. 2016 (WAC price reflects cost of Factor VIII replacement for an adult on prophylaxis)

3 *EPI Data from 2016 WFH Annual Survey;* NHF website: http://www.hemophilia.org/Bleeding-Disorders/Types-of-Bleeding-Disorders/Hemophilia-A;

Multiple Lifecycle Management Opportunities Exist to Capture Majority of 117K Hemophilia A Patients in our Territories

Goal to expand into additional Hemophilia A patient subpopulations over time



Valoctocogene Roxaparvovec
Initial label

Valoctocogene Roxaparvovec
Future label

BioMarin Production Capabilities for Valoctocogene Roxaparvovec

Expected to support 2000-3000 patients at launch

Process

- Have established a robust vector manufacturing process
- Consistent with ICH guidance facilitating global registration

Scale

- Fermentation, purification & filling operations performed at full scale

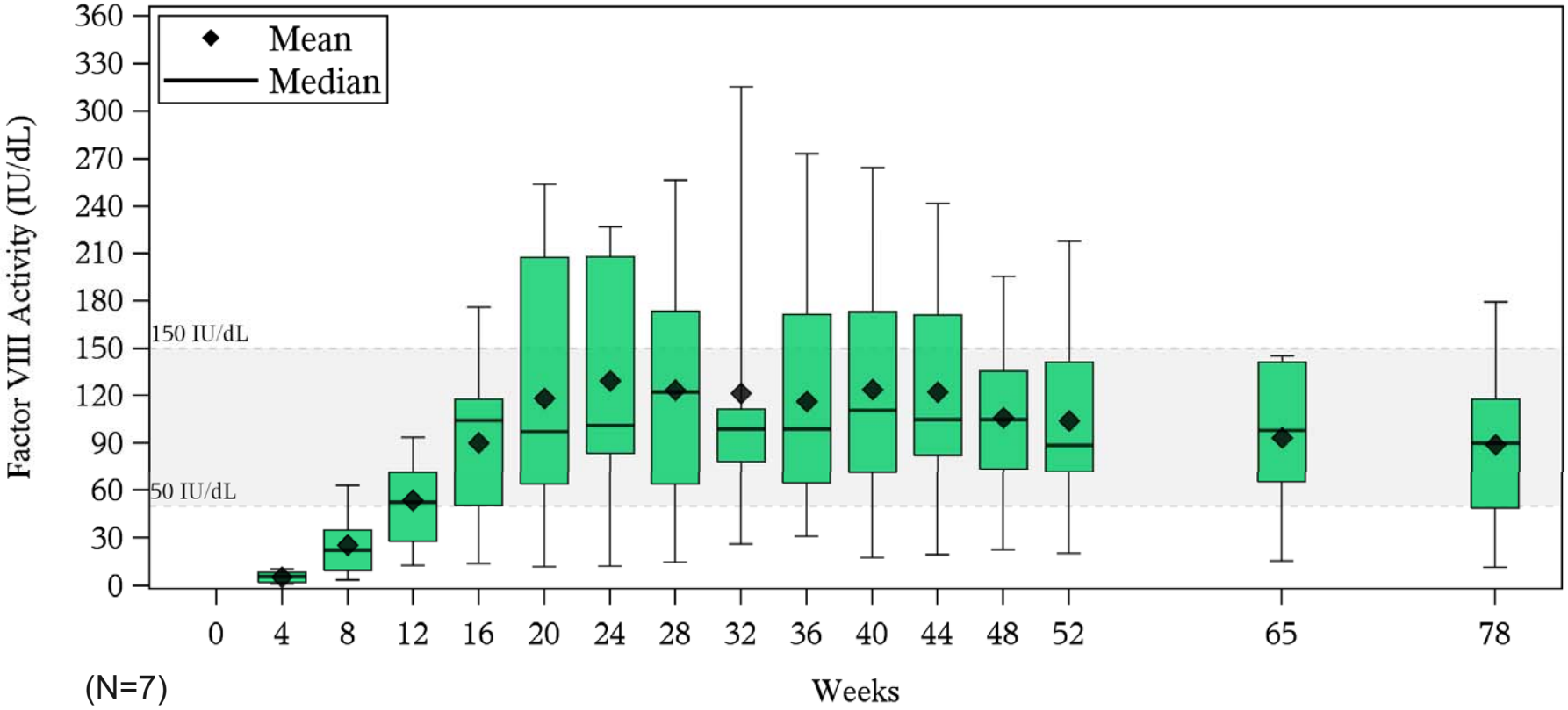
Facility

- Currently performing GMP production at commercial scale
- Material generated will support clinical and commercial demand

Published in NEJM: Sustainably Normalized through 1.5 Years

Baseline FVIII activity for all subjects at study start was ≤1% of normal level

Results from valoctocogene roxaparvovec 6e13 vg/kg cohort as presented at ASH, December 11, 2017



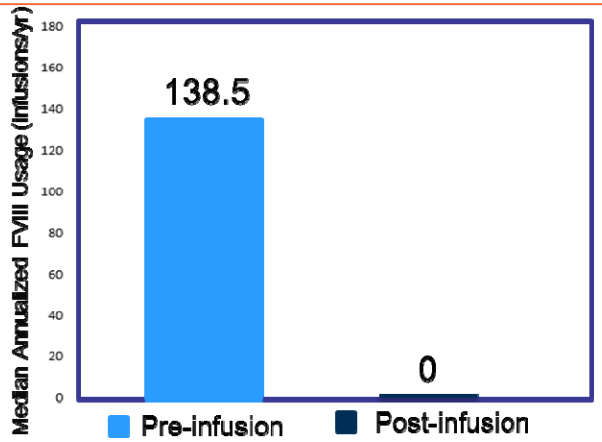
AAV5–Factor VIII Gene Transfer in Severe Hemophilia A

Savita Rangarajan, M.B., B.S., Liron Walsh, M.D., Will Lester, M.B., Ch.B., Ph.D., David Perry, M.D., Ph.D., Bella Madan, M.D., Michael Laffan, D.M., Hua Yu, Ph.D., Christian Vettermann, Ph.D., Glenn F. Pierce, M.D., Ph.D., Wing Y. Wong, M.D., and K. John Pasi, M.B., Ch.B., Ph.D.

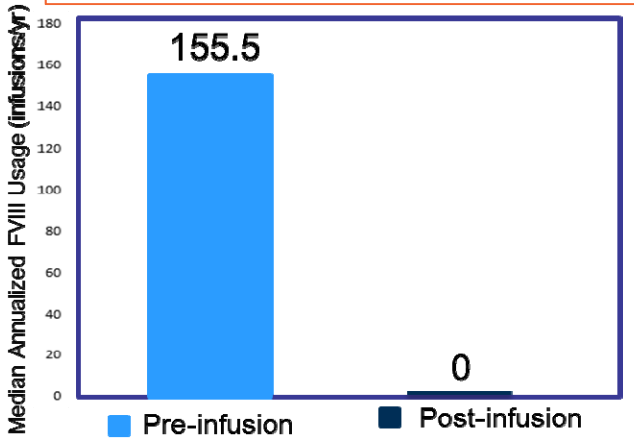
N Engl J Med 2017; 377:2519-2530, December 28, 2017, DOI: 10.1056/NEJMoa1708483

Annualized FVIII Usage and ABRs Reduced To Zero Post Infusion with Valoctocogene Roxaparvovec after FVIII > 5%

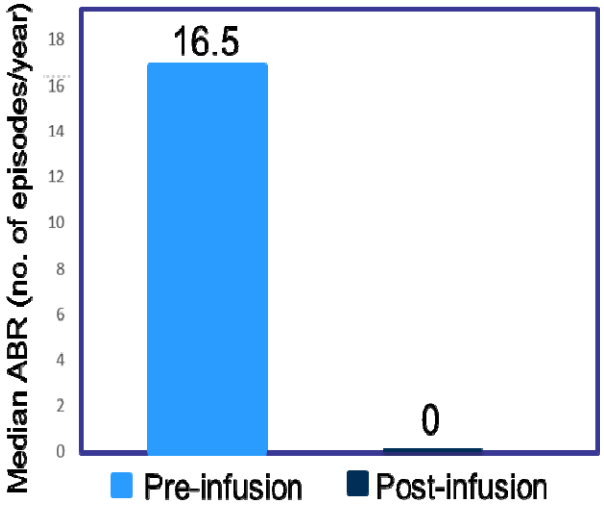
FVIII use Pre- and Post-Infusion 6E13 dose



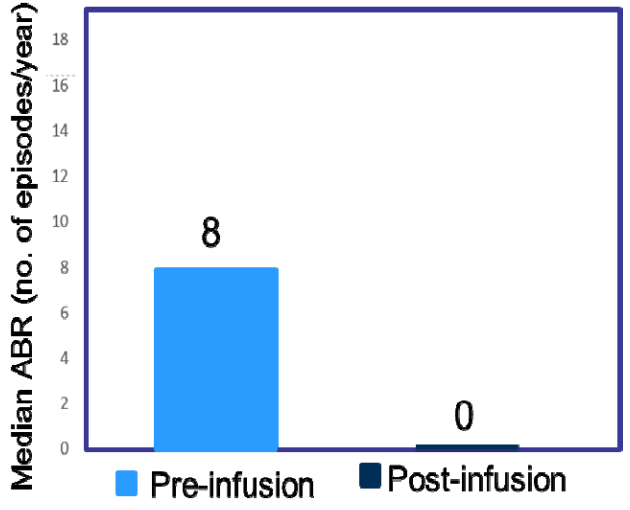
FVIII use Pre- and Post-Infusion 4E13 dose



ABRs Pre- and Post-Infusion 6E13 dose



ABRs Pre- and Post-Infusion 4E13 dose



Enthusiasm is Building for Use of Gene Therapy to Treat Hemophilia A

Market research indicates high likelihood of adoption

- 76% of respondents indicated FVIII activity over 50% would qualify as a transformational benefit that would make gene therapy the treatment of choice

- ~30-50% of adult Hemophilia A patients expected to be treated with gene therapy 3-6 months following approval

"….I find it amazing……It would mean that we would manage a situation where the patient can produce their own FVIII. That would be magic for patients." - France, HTC Nurse

Results of physician survey from Trinity Market Research and BioMarin Market Research

Unparalled Development Program and Manufacturing Expertise with Valoctocogene Roxaparvovec

First GENEr8 study expected to complete enrolment by year-end 2018

Global Phase 3 programs GENEr8-1 (6e13) and GENEr8-2 (4e13)

- 38 sites identified worldwide in 10 countries; N=40 each study; 52 weeks
- Patient enrolment began 4Q17
- Enrolling adults with severe hemophilia A who received FVIII prophylaxis

Phase 1/2 Study in AAV5+ with 6e13 kg/vg dose

- N=~10
- Two cohorts: Titer $\leq$ 500 and Titer > 500
- First patient expected to enroll in 1H18

BioMarin facility to supply pivotal programs in early 2018

- Same site for Phase 3 and commercialization

BIOMARIN

Vimizim

elosulfase alfa

Indication:

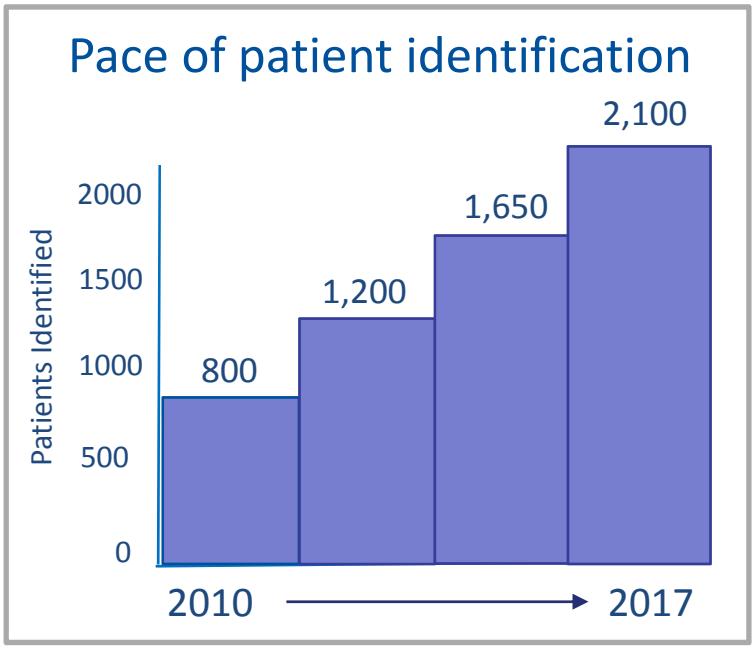
Morquio A Syndrome (MPS IVA)

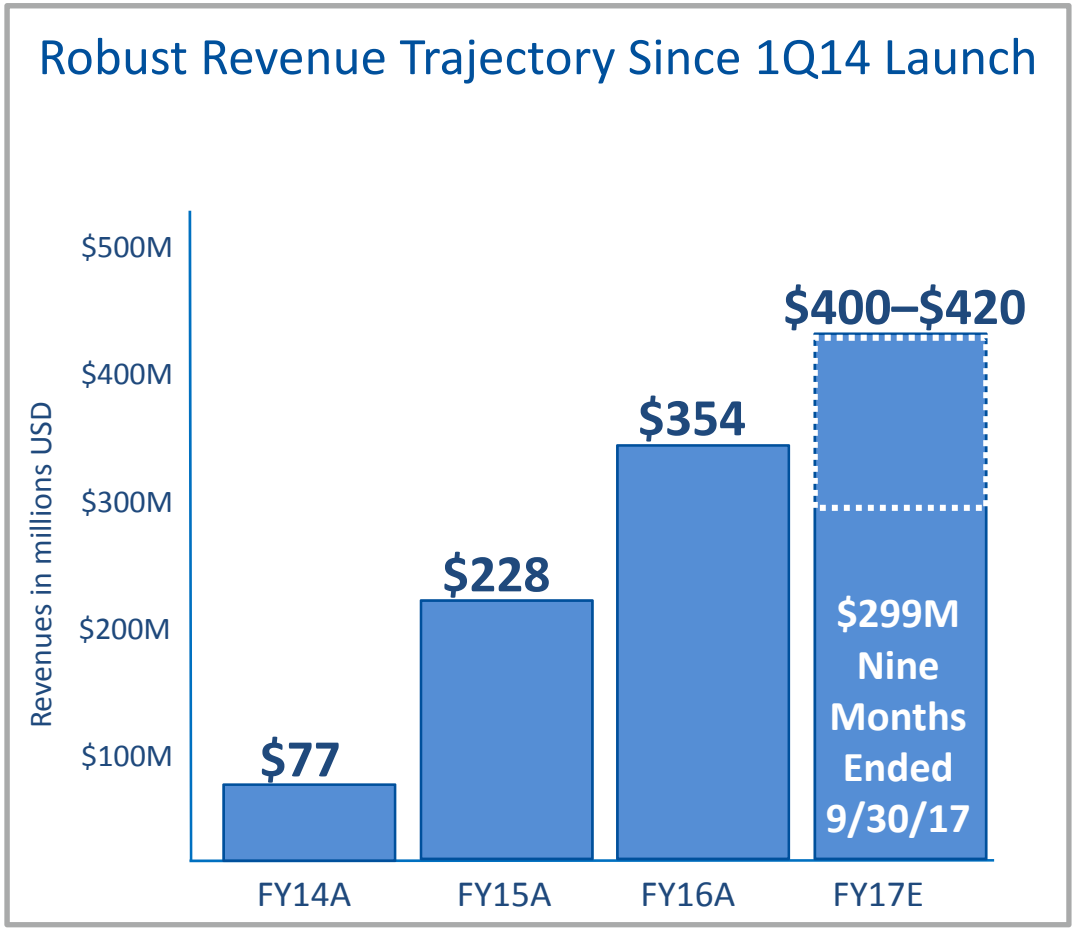
Vimizim FY 2017 Revenue Guidance $400M-$420M

Potential $1B market opportunity based on epidemiology

2100 patients identified

- New patient identification continues
- Epidemiology suggests 3000 WW





Four Pillars of Growth Driving Value

Strong and Growing Base Business:
YTD 2017 Revenues Increased 16% Year over Year

New Product Launches:
Brineura underway in US/EU; Pegvaliase potentially 2018

Potential $1 Billion Opportunities on the Horizon:
Vosoritide and Valrox

Turning the Corner Towards Profitability in 2017:
Expect FY non-GAAP Income

BIOMARIN

Multiple Portfolio Events Over the Next 12 months

Valoctocogene Roxaparvovec for Hemophilia A	Phase 3 enrollment start	4Q17
	Phase 3 material from BMRN plant	1H18
	Phase 2 one year update with 4e13 dose	Mid-2018
	Phase 2 two year update with 6e13 dose	Mid-2018
	First Phase 3 enrollment completion	YE 2018
Pegvaliase	MAA submission to EU	1Q18
	PDUFA	May 2018
	Potential US launch (if approved at PDUFA)	2H18
Vosoritide	Phase 3 enrollment completion	Mid-2018
	Phase 2 Infant/toddler study enrollment	1H18
BMN 250 for MPS IIIB	Phase 1/2 study enrollment continues	2018
BMN 290 for Freidriech's ataxia	IND submission/Phase 1/2 study start	2H18

THANK YOU

BIOMARIN