

# Bioverativ Investor Day

January 6<sup>th</sup>, 2017

# Forward-Looking Statements

- This presentation contains forward-looking statements, including statements relating to: the planned separation of Bioverativ from Biogen; business and strategic objectives; growth prospects and potential opportunities for commercial products and pipeline programs; planned geographic expansion; manufacturing, supply and distribution arrangements; relationships with collaborators and other third parties; research and development activities and priorities; anticipated clinical trials and data readouts; business development plans and opportunities; and expected capitalization, revenues, operating margin, cash flows and other financial guidance. These forward-looking statements may be accompanied by such words as “anticipate,” “believe,” “could,” “estimate,” “expect,” “forecast,” “intend,” “may,” “plan,” “potential,” “project,” “target,” “will” and other words and terms of similar meaning. You should not place undue reliance on these statements.
- These forward-looking statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including: Bioverativ’s dependence on revenues from sales of ELOCTATE and ALPROLIX; failure to compete effectively due to significant product competition in the markets in which Bioverativ operates; product quality or safety concerns, including the occurrence of adverse safety events; product development risks; risks associated with clinical trials; risks relating to actions of regulatory authorities; risks related to reliance on third parties for manufacturing, supply and distribution of Bioverativ’s products and product candidates; difficulties in obtaining and maintaining adequate coverage, pricing and reimbursement for Bioverativ’s products; failure to obtain and maintain adequate protection for intellectual property and other proprietary rights; risks of doing business in international markets; risks associated with current and potential future healthcare reforms; failure to identify and execute on business development and research and development opportunities; Bioverativ’s dependence on relationships with collaborators and other third parties for revenue and other aspects of its business; loss of key employees or inability to attract and retain key personnel; disruptions to, or other adverse impact on Bioverativ’s relationships with its customers and other business partners; failure to comply with legal and regulatory requirements affecting Bioverativ’s business; the impact of global economic conditions; fluctuations in foreign exchange and interest rates; changes in the law concerning the taxation of income; risks relating to technology failures or breaches; the outcome of any significant legal proceedings; the adequacy of the Bioverativ’s cash flows from operations; Bioverativ’s lack of operating history as a standalone business; risks relating to the separation from Biogen, including, among others, risks that the separation will be completed in a timely manner or at all, failure to achieve the anticipated benefits from the separation, reliance on Biogen and other third parties to provide certain services post-separation, restrictions to preserve the tax-free treatment of the separation that may impact Bioverativ’s strategic and operating flexibility, and Bioverativ’s ability to satisfy liabilities and potential indemnification obligations in connection with the separation; and other risks and uncertainties described in the Risk Factors section of Bioverativ’s Registration Statement on Form 10 and other filings with the Securities and Exchange Commission.
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# Today's Agenda

Time	Topic	Speaker
10:05-10:10am	Introduction	<b>Paul Clancy</b> <i>Biogen CFO</i>
10:10-10:50am	Investment opportunity and strategic vision	<b>John Cox</b> <i>Chief Executive Officer</i>
10:50-11:10am	Global Therapeutic Operations	<b>Rogério Vivaldi, MD, MBA</b> <i>Chief Global Therapeutic Operations Officer</i>
11:10-11:20am	Financial overview	<b>John Greene</b> <i>Chief Financial Officer</i>
11:20-11:25am	Closing	<b>John Cox</b> <i>Chief Executive Officer</i>
11:25-11:50am	Break	
11:50-12:40pm	Q&A	Bioverativ Leadership Team



**Welcome**

Paul Clancy, Biogen CFO

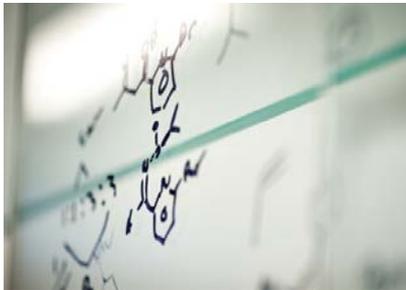


**Investment opportunity and strategic vision**

John Cox, CEO

*Our Vision is to become the...*

*leading hematology rare disease company  
committed to creating significant progress  
for patients*





# A Unique and Compelling Investment Opportunity



Integrated capabilities

Talented team

Strong hemophilia franchise

Capitalized to create value

# Bioverativ: Why Compelling?



Expected revenues of >\$800 million in 2016 targeting a \$10B+ market in hemophilia



Generated \$203 million of net cash flows from operations from Q1-Q3 2016



Geographic presence in U.S., Japan, and Canada with opportunities to expand



R&D, clinical development, regulatory prowess in hematology



Proven commercial execution



Expertise in process development, manufacturing, and supply of complex biologics



Strategic Business Development and on-going R&D are levers for growth



Appropriately sized organization

# Positioned for Growth as a Stand Alone Company

*Maximize  
Potential of  
ELOCTATE &  
ALPROLIX*

**Rapidly  
Advance our  
Discovery  
Molecules**

**Pursue  
Strategic  
Opportunities**

# Accomplished and Driven Executive Leadership Team



**John Cox**  
*Chief Executive Officer*



**John Greene**  
*Chief Financial Officer*



**Rogerio Vivaldi, MD, MBA**  
*Chief Global Therapeutic  
Operations Officer*



**Richard Brudnick**  
*EVP of BD & Alliance  
Management*



**Lucia Celona**  
*Chief HR & Corporate  
Communications Officer*



**Andrea DiFabio**  
*Chief Legal Officer*

# Strong Scientific, Medical Leaders with Hematology Expertise



**Rob Peters, Ph.D.**  
**SVP, Research**

16+ years experience.  
Renowned hemophilia scientist.  
Inventor of Fc fusion technology  
at Syntonix



**Maha Radhakrishnan, M.D.**  
**SVP, Medical**

12+ years in Medical Affairs leadership  
roles at BMS, Cephalon and Biogen.  
Global experience, most recently Europe  
Medical Head



**Nisha Jain, M.D.**  
**Executive Director, Medical**

16+ years experience in hematology and  
rare diseases including experience in NIH  
and FDA



**Bill Hobbs II, M.D., Ph.D.**  
**Executive Director,  
Clinical Development**

10+ years of clinical and  
research experience. A leading  
treater of SCD and administered  
only adult SCD program in  
Pacific NW



**Michael Poirier, M.S.**  
**SVP, Regulatory & Safety**

16+ years in Regulatory Affairs at Biogen;  
Global Regulatory lead on key programs  
including Avonex, Tysabri, Tecfidera, and  
most recently Spinraza

# A Unique and Compelling Investment Opportunity

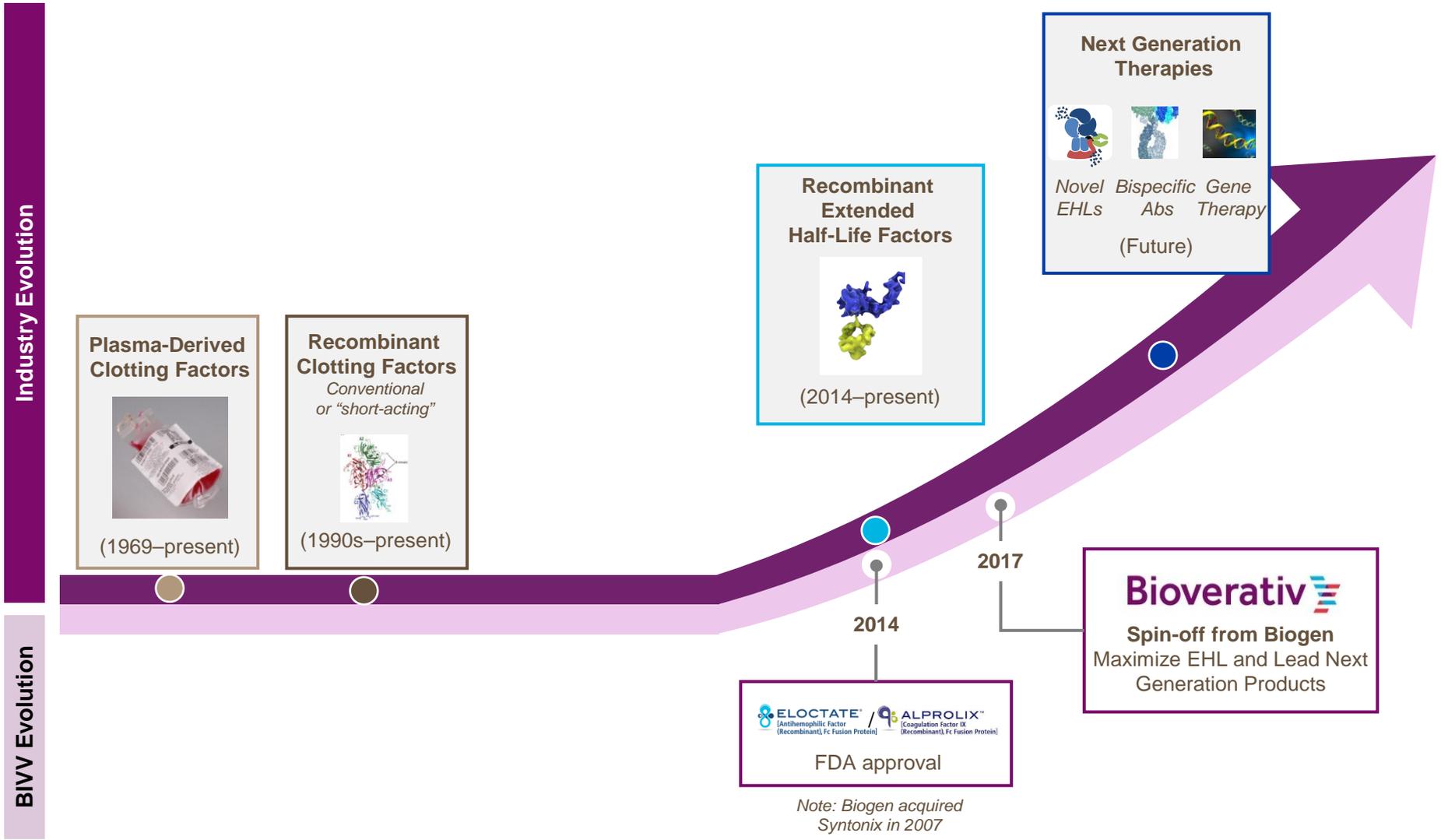
Integrated capabilities

Talented team

**Strong Hemophilia Franchise**

Capitalized to create value

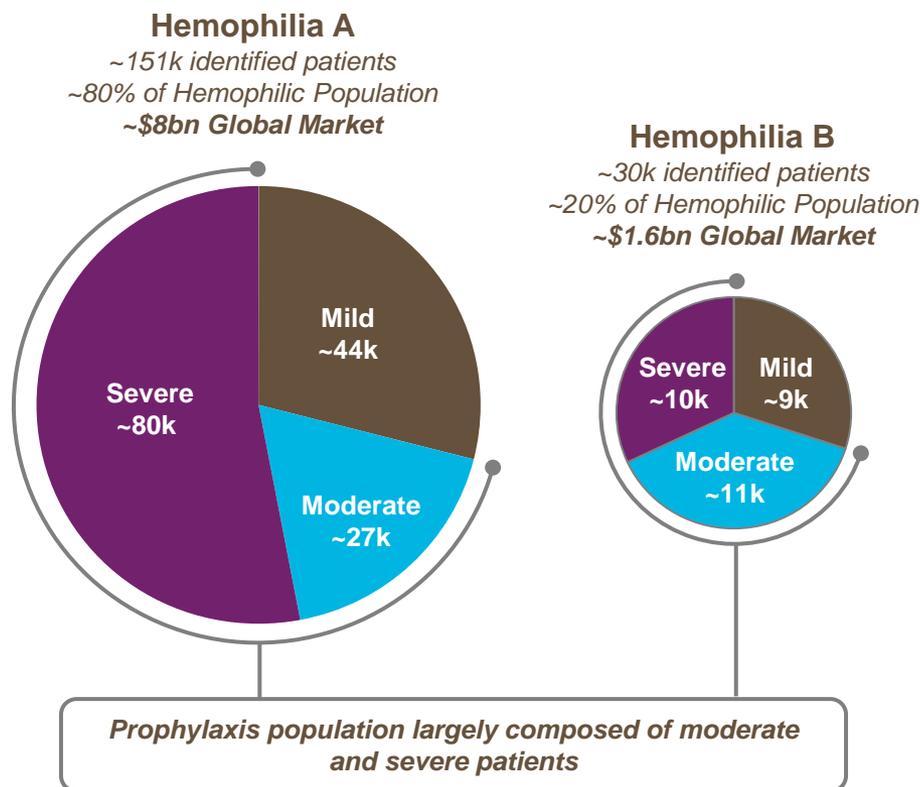
# Our Therapies; First Innovation in 20 Years



# Hemophilia is a \$10B+ Global Market, Growing at 7%

Opportunity for growth within each market segment

## Hemophilia A&B Factor Market: ~\$10B Globally



Note that the total estimated population with hemophilia is larger at ~400k estimated patients versus ~181k identified patients

## Growing Hemophilia Market:

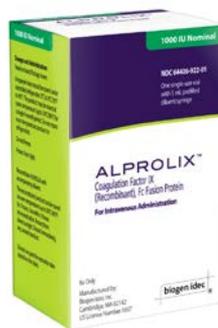
- Prevalent population increasing globally
- Patients shifting from **short-acting to EHL factors** and from **on demand to prophylaxis treatment**
- ~30% of Hem A pts will develop inhibitors
- ~10% of Factor units used for immune tolerance induction (ITI) to eradicate inhibitors
- Bypassing agents for inhibitor patients expected to generate ~\$2.1B in 2016

Sources: WFH 2016, MRB 2016, AHTN 2016, Evaluate Pharma

# ELOCTATE & ALPROLIX Have Delivered Strong Uptake Since Launch and are Approaching \$800M+ in 2016

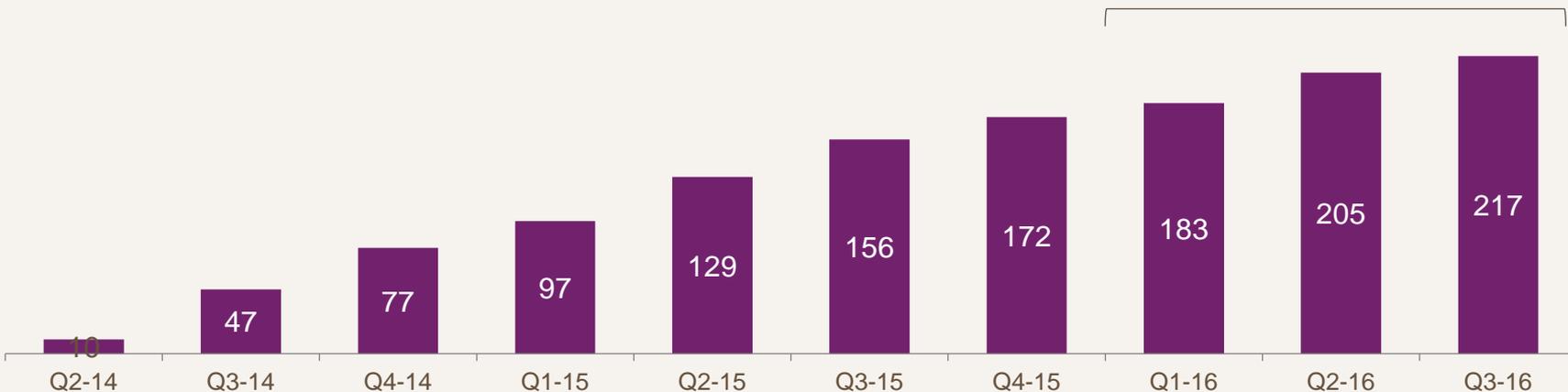


**ELOCTATE®**  
[Antihemophilic Factor  
(Recombinant), Fc Fusion Protein]

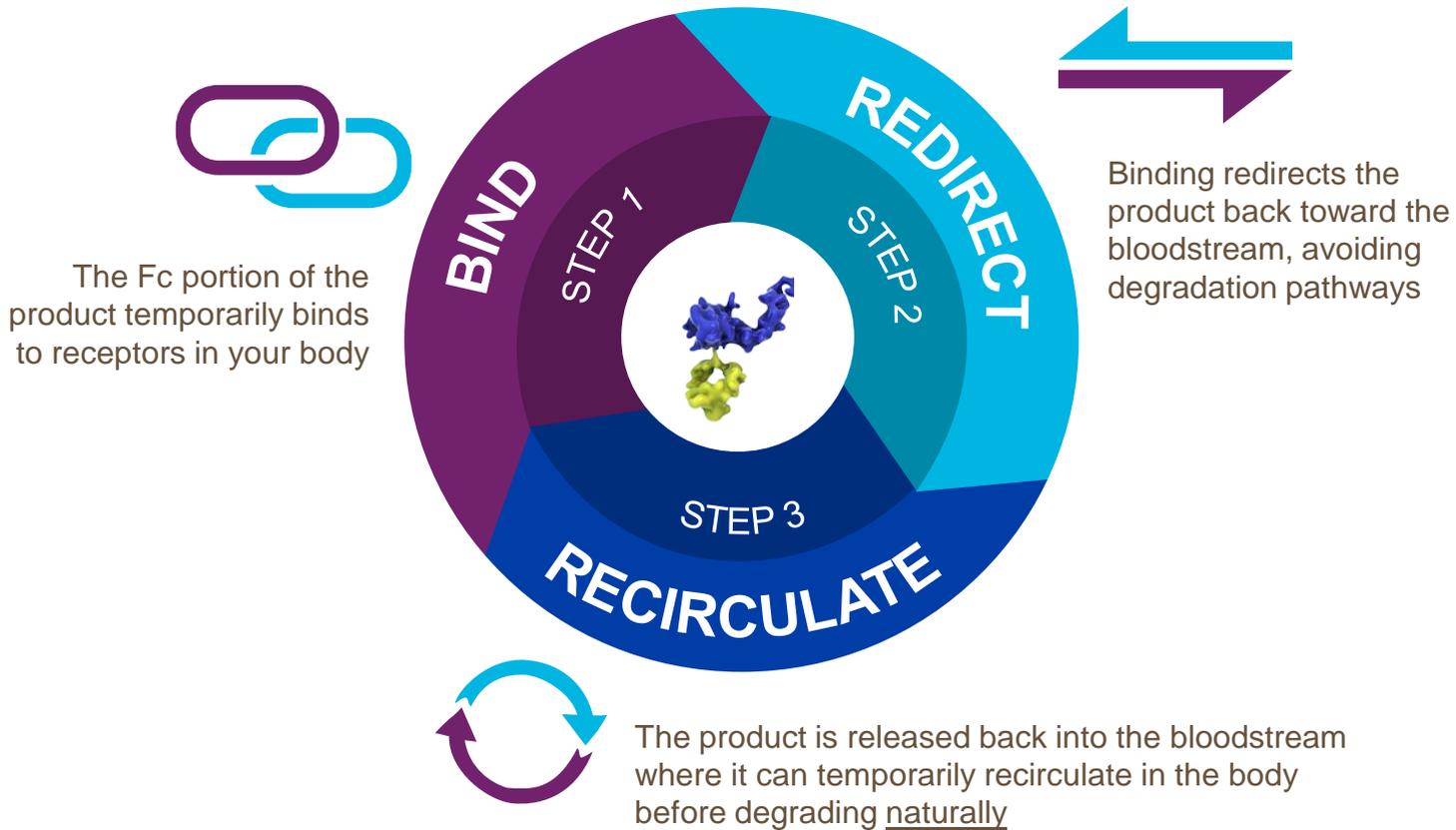


**ALPROLIX®**  
[Coagulation Factor IX  
(Recombinant), Fc Fusion Protein]

Hemophilia Product Revenue (\$mm)



# ELOCTATE and ALPROLIX are the Only Factors Utilizing Proprietary Fc Fusion Technology



# Potential Opportunities to Address Significant Unmet Medical Needs with ELOCTATE and ALPROLIX

## Immune Tolerance Induction

- Factor replacement can eradicate inhibitors vs bypassing them
- Based on early case reports<sup>(1)</sup>, further examination of ELOCTATE's potential for ITI is warranted

## Joint Health

- Risk of progressive and extremely debilitating joint disease is substantial
- Planning to further investigate ELOCTATE and ALPROLIX impact on joint health

## Women with Hemophilia

- Many women with recessive gene for hemophilia have bleeding problems
- Need to better understand impact of bleeding problems on women and opportunities to mitigate their symptoms

<sup>(1)</sup> Malec *et al* Haemophilia 2016, Ragni *et al* ASH 2016, Malec *et al* Blood 2015

# A Unique and Compelling Investment Opportunity

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Strong Hemophilia Franchise

Capitalized to create value

# Pipeline of Novel, Next Generation Hemophilia, Beta-Thalassemia, and Sickle Cell Disease Candidates

Drug	Indication	Description	Modality	Discovery	Preclinical	Clinical	Marketed
BIVV 001 <i>rFVIII<sup>Fc</sup>-VWF-XTEN</i>	Hem A	EHL factor <b>1x/weekly dosing or less frequent</b>	Biologic	[Progress bar]			
BIVV 002 <i>rFIX<sup>Fc</sup>-XTEN</i>	Hem B	EHL factor <b>Subcutaneous</b>	Biologic	[Progress bar]			
Sangamo collaboration	Beta Thal- assemia	Zinc finger nuclease (ZFN)	Genome Editing	[Progress bar]			
	Sickle Cell	Zinc finger nuclease (ZFN)	Genome Editing	[Progress bar]			
San Raffaele collaboration	Hem A	Lentiviral vector	Gene Therapy	[Progress bar]			
	Hem B	Lentiviral vector	Gene Therapy	[Progress bar]			
FVIIIa mimetic bispecific ab	Hem A; Inhibitors	MOA not disclosed	Biologic	[Progress bar]			
Multiple early stage programs	Sickle Cell	Multiple MOAs	Sm Molecules	[Progress bar]			

XTEN licensed from Amunix

# BIVV 001 Designed to Extend Hemophilia A Prophylaxis to Once Weekly or Less Frequently

*rFVIII<sub>FC</sub>-VWF-XTEN*

## Technology

Uniquely engineered factor VIII molecule with a region of Fc dimer, VWF, and XTEN polypeptides

- Fc monomer, like Eloctate, enables recycling to extend time in circulation
- D'D3 inhibits binding to VWF which limits the ceiling for current FVIII products
- XTEN insertions increase half-life by protecting from clearance/ proteolysis

## Potential Clinical Profile

Trials will be designed to test potential for prophylaxis intervals in Hemophilia A of once weekly or less frequent dosing

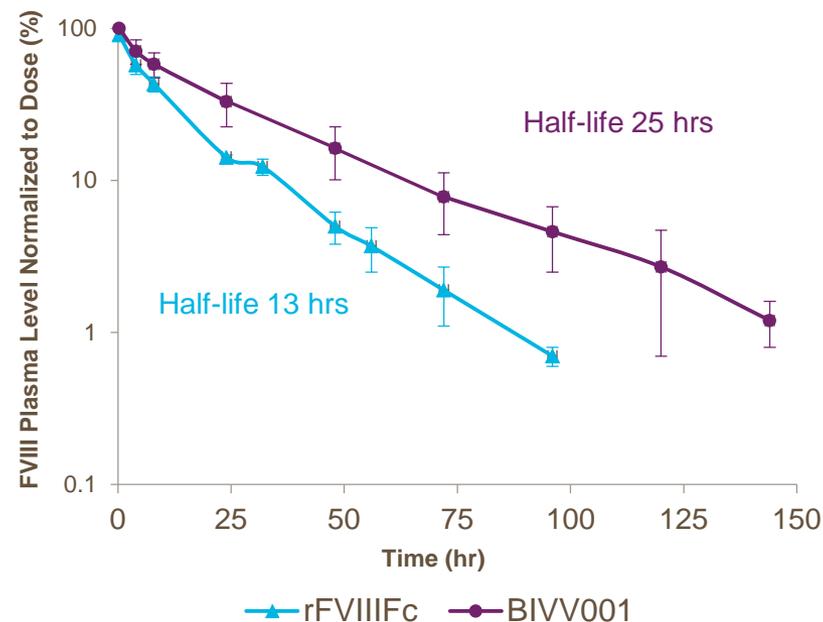
## Competitive Positioning

rFVIII molecule with potential to eliminate 1/2 life limitations found with other EHL products

## Timing

Intend to move into the clinic in 2017

## Improved PK Profile of Intravenously Delivered BIVV 001 in Cynomolgus Monkeys



***BIVV 001 showed 2-fold improvement in pharmacokinetic property compared to rFVIII<sub>FC</sub> in cyno monkeys***

Note: BIVV 001 is currently BIIB073, XTEN technology licensed from Amunix

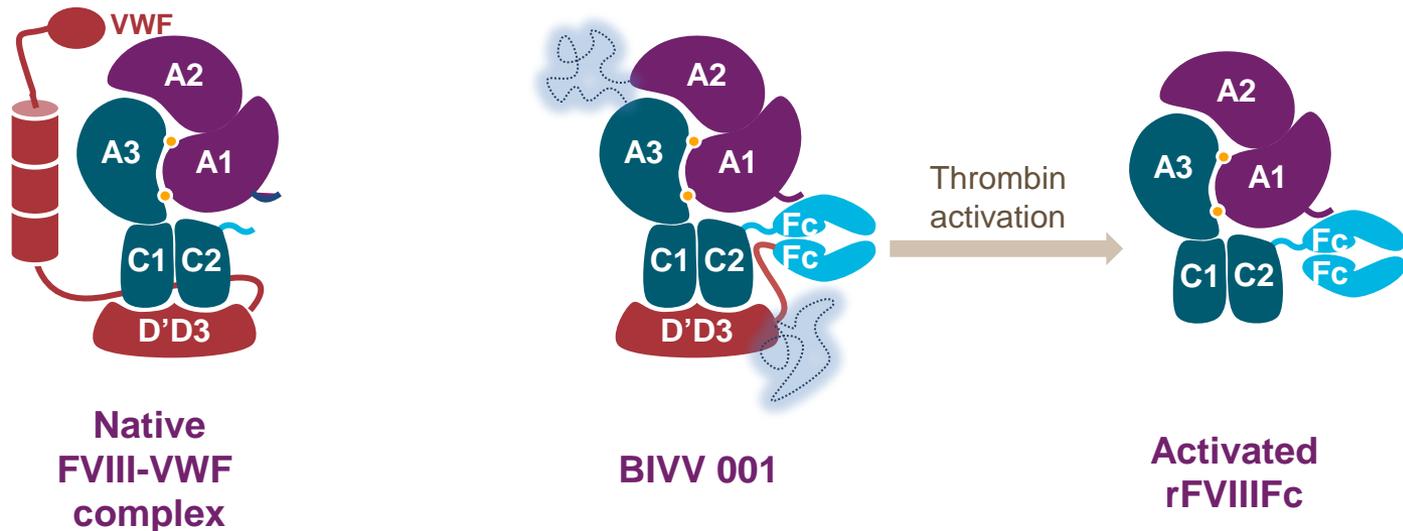
# Scientific Rationale for BIVV 001 Molecule Design

Novel fusion protein, consisting of:

**D'D3 domains of VWF** provide protection & stability of VWF while **evading half-life limitation of endogenous VWF**

**XTEN polypeptides**, which improve the pharmacokinetic profile and degrade naturally

**rFVIII fused to dimeric Fc** which maintains thrombin-mediated release of FVIII from VWF like natural FVIII. Once released FVIII will then bind phospholipids and participate in the clotting cascade



# BIVV 002 Designed to Enable Subcutaneous Administration

## rFIXFc-XTEN

### Technology

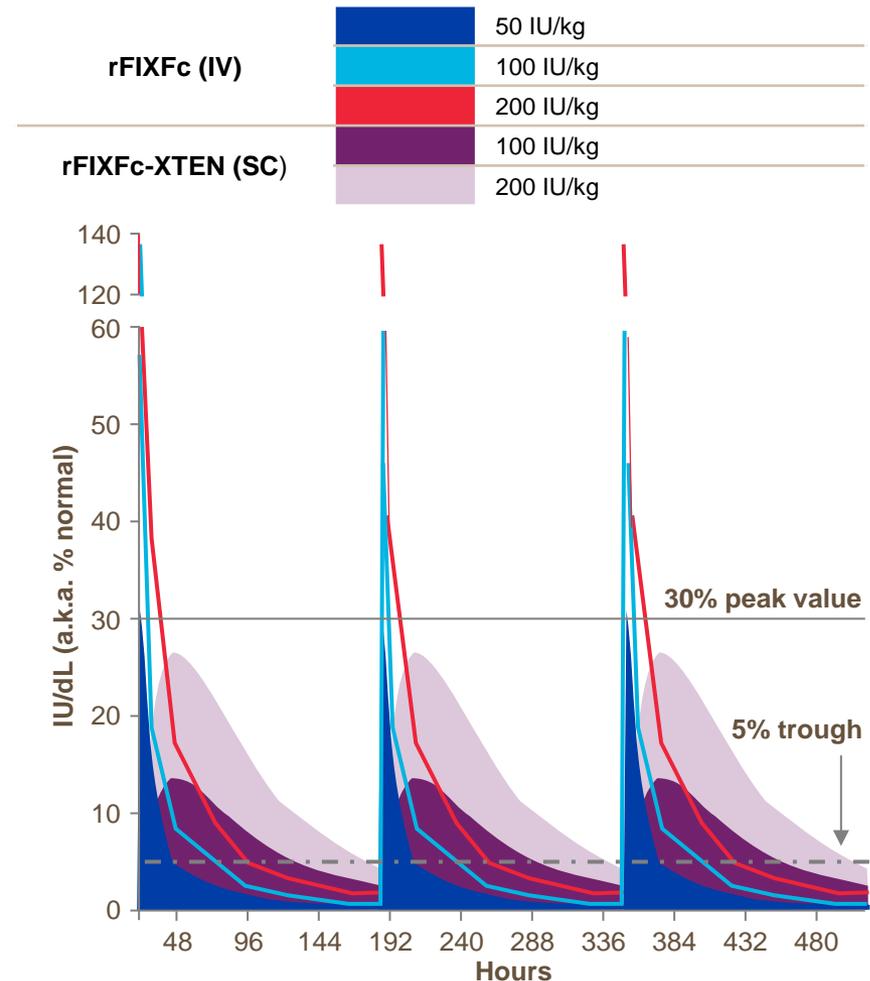
Combines Fc dimer and XTEN technology along with R338L Padua Factor IX variant in the treatment of Hemophilia B

### Potential Clinical Profile

Trials will be designed to explore potential for subcutaneous dosing. Leverages Fc fusion technology

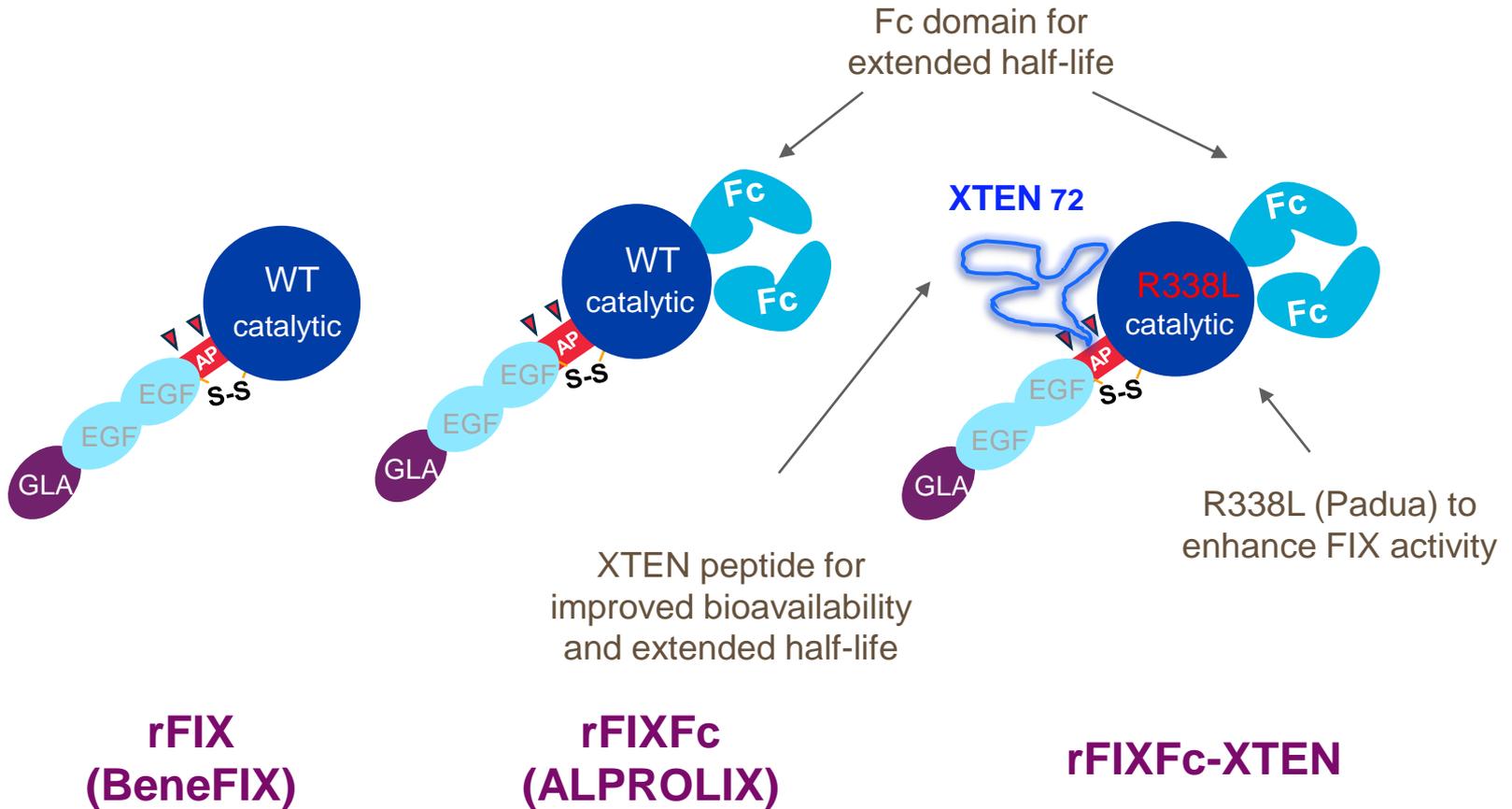
### Competitive Positioning

Potential for subcutaneous dosing could lessen burden of care and to compete with gene therapy



Note: BIVV 002 is currently BIIB085, XTEN technology licensed from Amunix  
 Source: Preliminary modeling Arjan van der Flier & Qin Weng, DMPK

# Molecular Design of BIVV 002



*Activation of FIX cleaves and releases the AP domain and attached XTEN so that the resulting active FIXFc molecules are identical (except for R388L)*

# Collaboration on Sangamo's Non-viral, Ex-vivo ZFN-mediated Genome Editing Programs for $\beta$ -thal, SCD

## Technology

Zinc finger nuclease (ZFN)-mediated genome editing program for beta-thalassemia and sickle cell disease is based on the use of genome editing technology to modify a patient's own (autologous) hematopoietic stem progenitor cells (HSPCs)

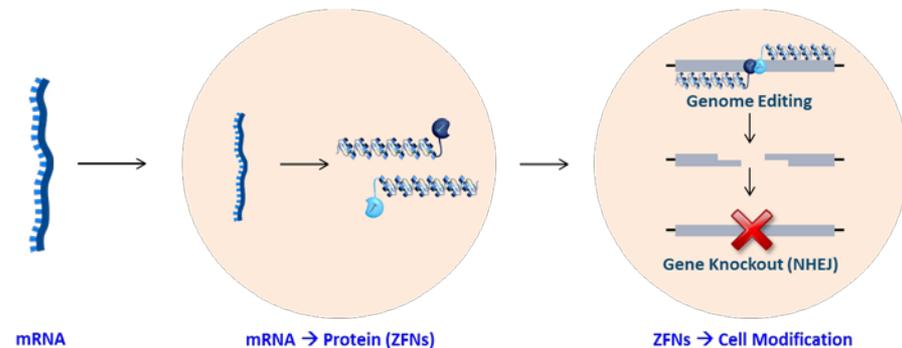
## Potential Clinical Profile

Trials will explore potential in both beta-thalassemia and sickle cell disease, diseases with significant unmet medical need

## Competitive Positioning

Potential gene therapy treatment for rare diseases with significant unmet need

## ZFN-mediated Enhancer Knockout



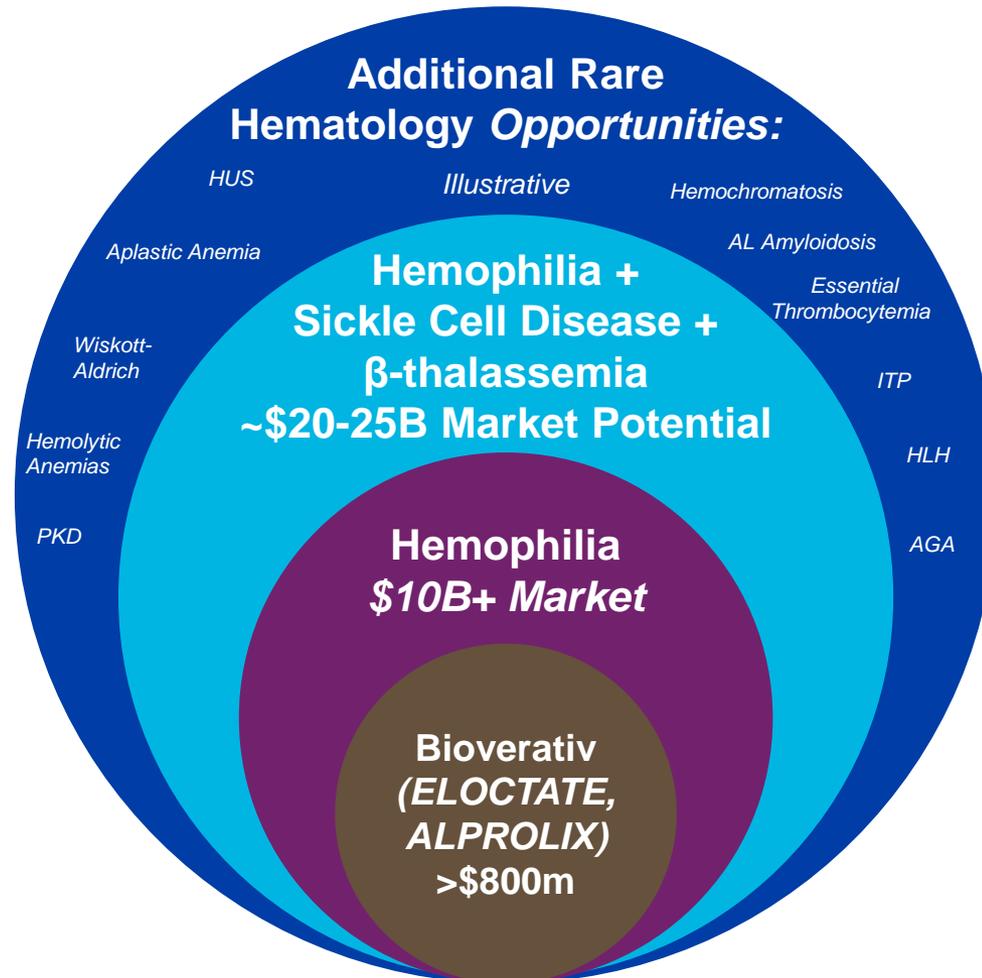
MOA hypothesis in  $\beta$ -thal: increase HbF to compensate for no or low  $\beta$ -globin levels allowing for more normal RBC production and RBC lifespan

MOA hypothesis in SCD: increase HbF levels to dilute the HbS, block polymerization, allow for more normal RBC function, and decrease RBC destruction (hemolysis)

***$\beta$ -thal and SCD are rare diseases with significant unmet medical needs and are priority areas of focus for our Hematology franchise***

# Large Opportunity Exists for Expansion in Rare Hematology

## Global Non-Malignant Hematology Market (2016)



Source: EvaluatePharma, Decision Resources, Company management

# Business Development Vision: Sector Expert and Partner of Choice

- Numerous rare hematologic diseases with high unmet need and interesting accessible clinical stage assets
- Committed to exploring such opportunities to bolster our pipeline
- With our expertise we believe we can drive programs rapidly through the clinic and we aim to be the partner of choice
- Financial capacity provides potential to grow inorganically





## Global Therapeutic Operations

Rogério Vivaldi, MD, MBA

# Strategic Imperatives for Therapeutic Operations

## *Maximize*

*Potential of ELOCTATE  
and ALPROLIX*

## *Enhance*

*Value with Patient-Centric  
Approach*



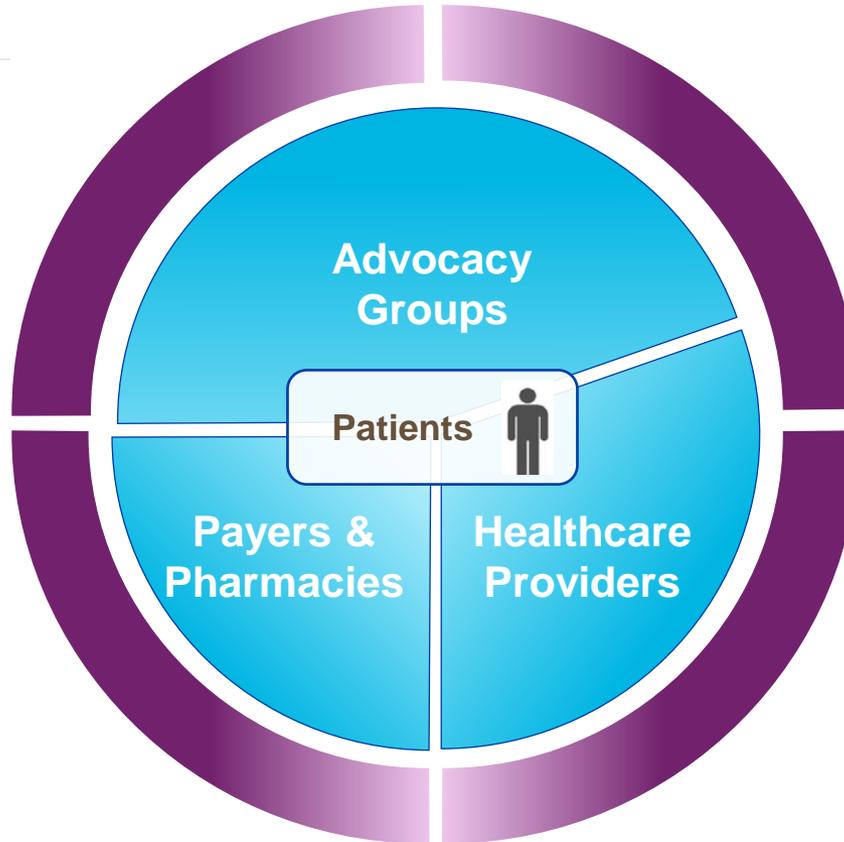
# Patient-Centric Model in Rare Hematology

## CoRe Managers

Work directly with the community to provide educational information, resources, and info about our programs and services

## National Account Managers

Dedicated to patient access working closely with payers



## Patient Services

Provides caregivers, patients and healthcare providers with dedicated and individualized support

## Account Executives

Provide HCPs and HTC's with fair and balanced information

***Bioverativ's commercial structure well-positioned to continue to serve the needs of all our hematology customers in an efficient way***

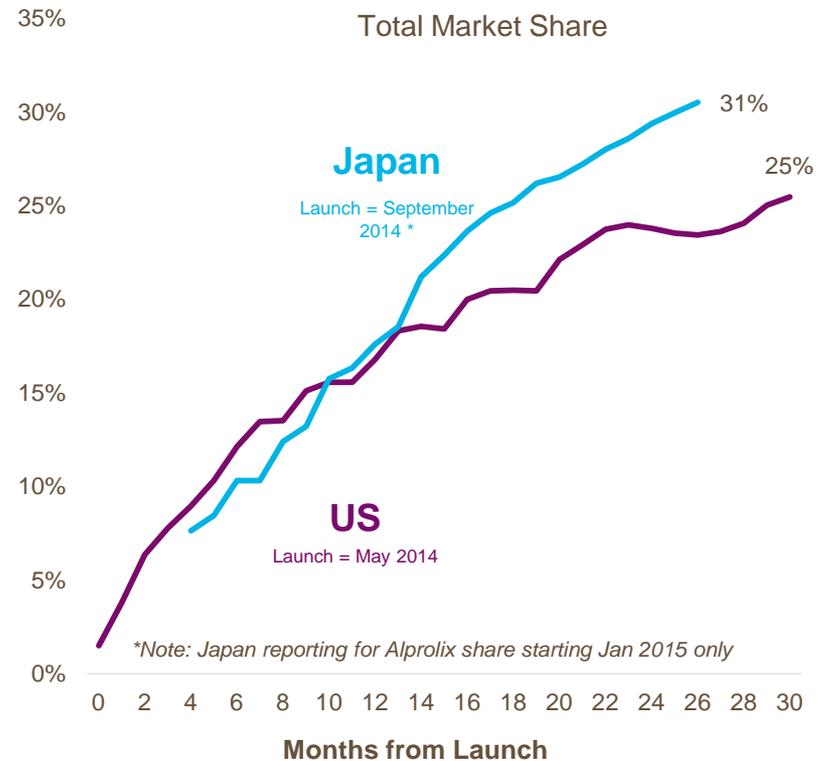
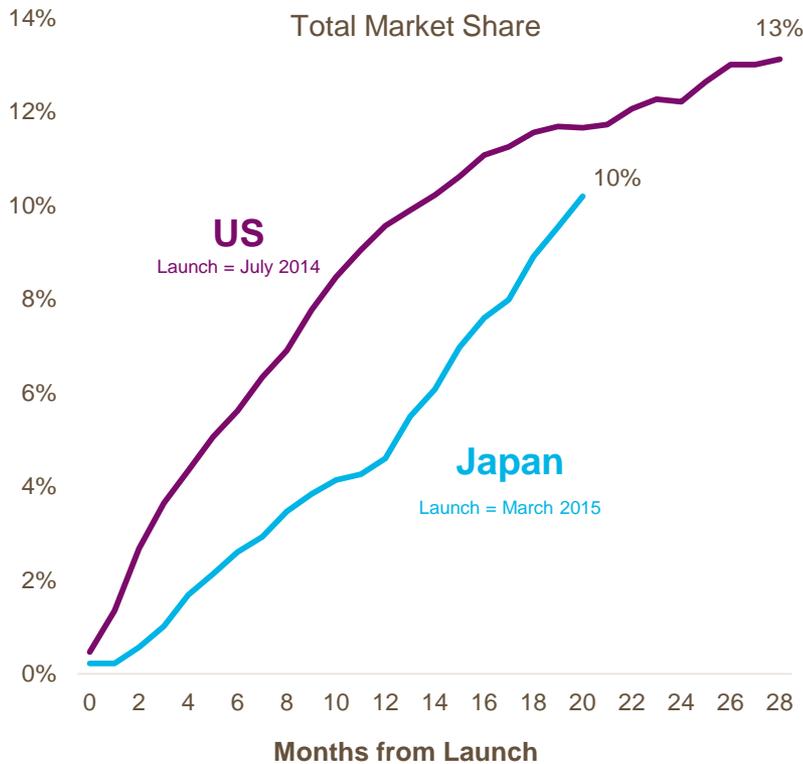
# Strong Launches Position ELOCTATE & ALPROLIX for Future Success



US launch Q2 2014, Japan Q1 2015, Canada Q1 2016



US launch Q2 2014, Japan Q3 2014, Canada Q4 2015



# Broad Reach Across Hemophilia Treatment Centers, Opportunity to Drive Further Depth of Prescribing

90%

of HTC\*s have PRESCRIBED ALPROLIX to AT LEAST 1 PATIENT

\*123 of 136 hemophilia treatment centers (HTCs)



**NUMBER OF UNITS DISTRIBUTED**

- 1,000,000+ units
- 400,000 – 1,000,000 units
- 550,000 – 400,000 units
- 0 – 50,000 units

90%

of HTC\*s have PRESCRIBED ELOCTATE to AT LEAST 1 PATIENT

\*123 of 136 hemophilia treatment centers (HTCs)



**NUMBER OF UNITS DISTRIBUTED**

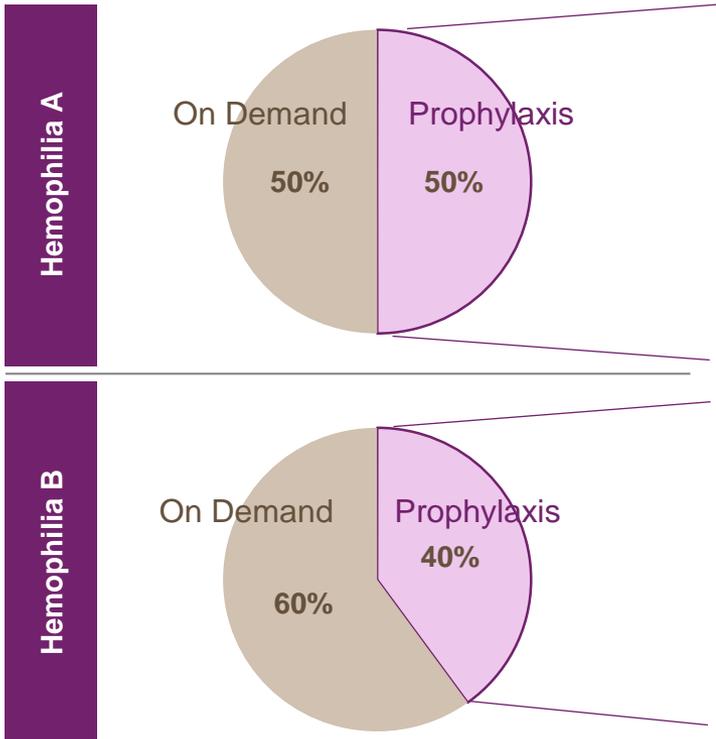
- 2,000,000+ units
- 750,000 – 2,000,000 units
- 250,000 – 750,000 units
- 0 – 250,000 units

Based on pharmacy dispensing records and HTC direct ordering through December 12, 2016.

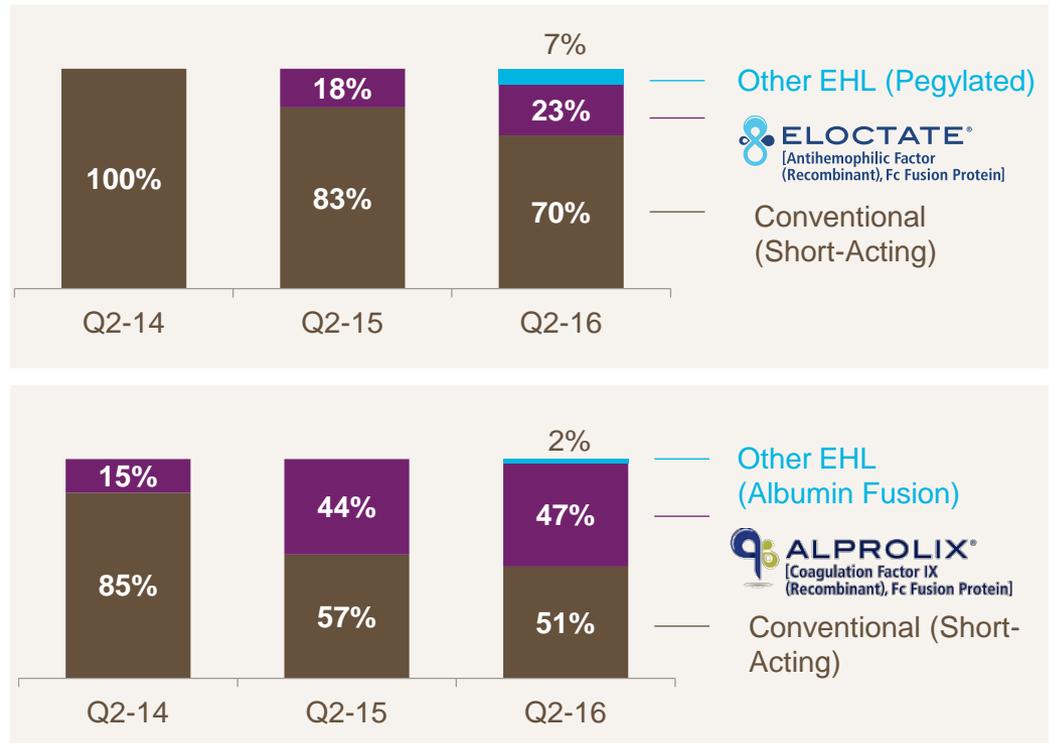
Source: Biogen. SPP data on file.

# Opportunity Remains for Further Growth from Shift to Prophylaxis and EHL Therapies

On Demand vs Prophylaxis

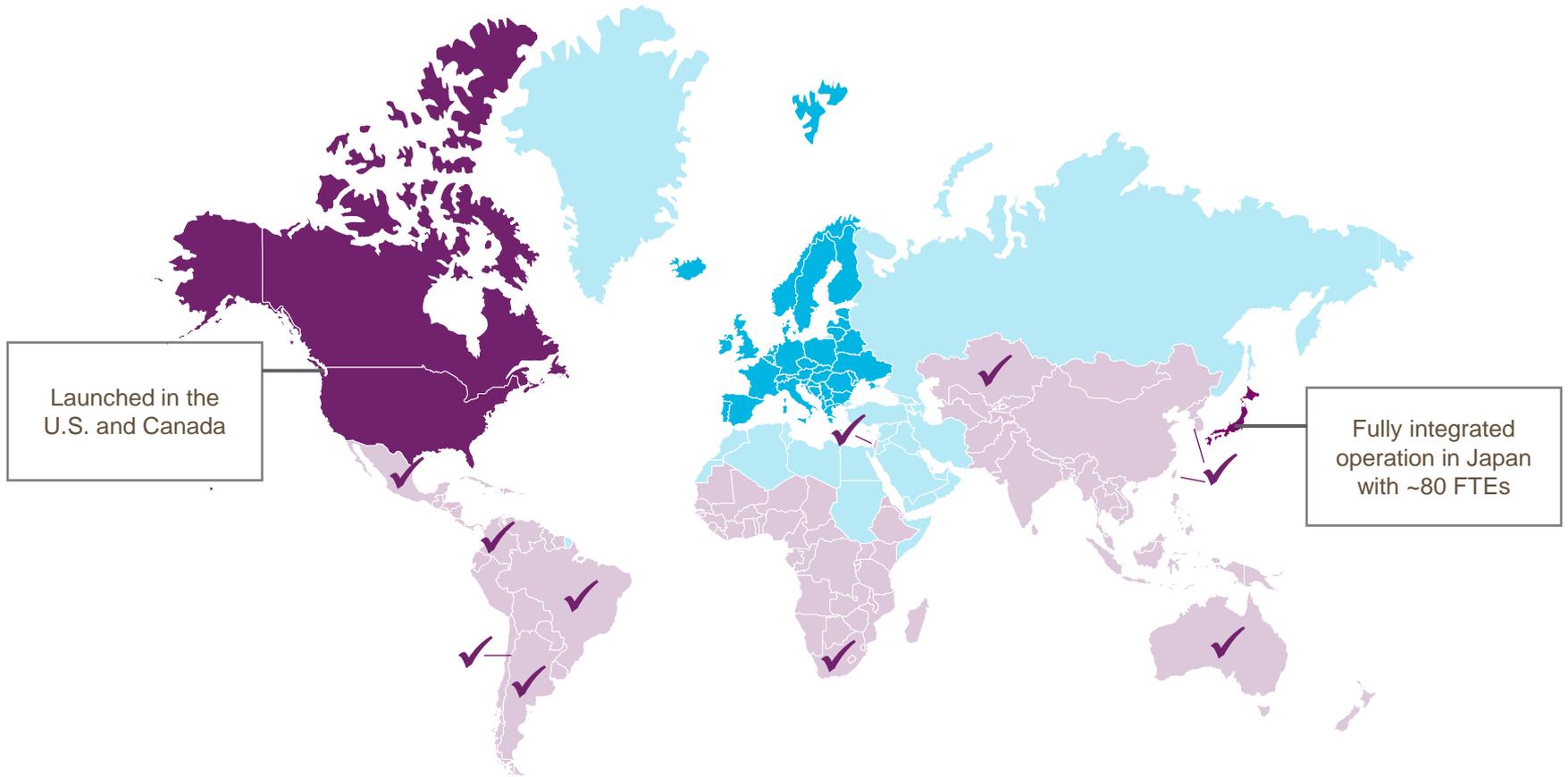


Market Share of Patients on Prophylaxis (U.S.)



Source: Q2 2016 US HTC Market Tracking FVIII and FIX Report

# Plans to Expand to New Markets Where Already Approved or Filed



✓ **BIVV approved / filings accepted**

**Bioverativ**

- ELOCTATE and ALPROLIX Approved
- Not-yet-commercialized Territory

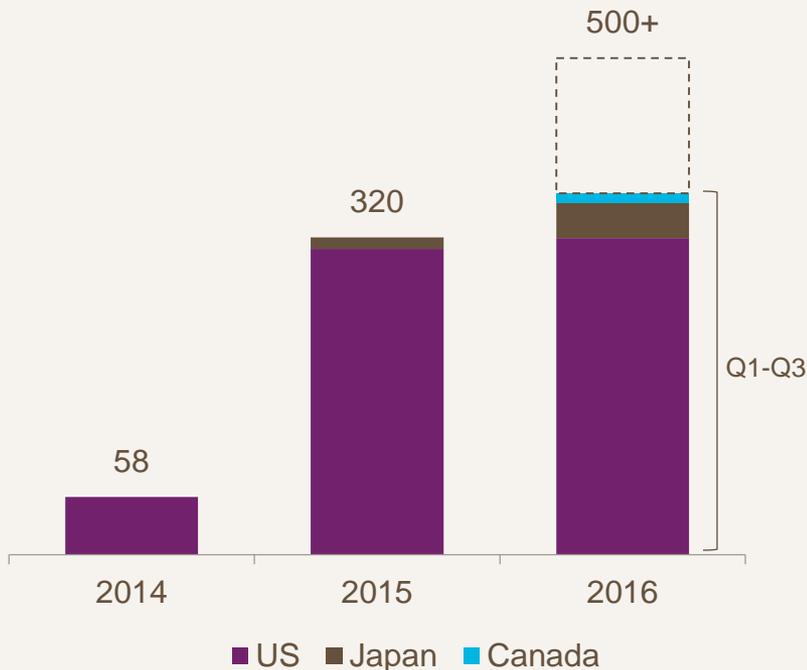
**sobi**

- ELOCTA and ALPROLIX Approved
- Not-yet-commercialized Territory

# ELOCTATE has Delivered Strong Performance and Growth Opportunities Remain



## ELOCTATE Revenue (\$M)



Approved for Hem A to reduce frequency of bleeding episodes with prophylactic infusions every 3 to 5 days

Advantage of more than two years of real-world experience and consistent long-term safety data

## Potential Growth Opportunities

- Continued shift to EHLs, and shift to prophylaxis
- Expansion into new geographies
- Increased patient access
- Treating women who have the recessive gene for hemophilia

Source: Investor Presentation, Company Website, Form 10

Note: Approved in EU in November 2015 under trade name Elocta®.

# Evolution of the Hemophilia A Landscape

## Near Term

## Longer Term

### Hemophilia A

- Competition from other EHL entrants
- Advancements in half-life extension technology including BIVV 001
- New MOAs including bispecific antibodies, RNAi therapeutics, gene therapies, etc.

### Inhibitors

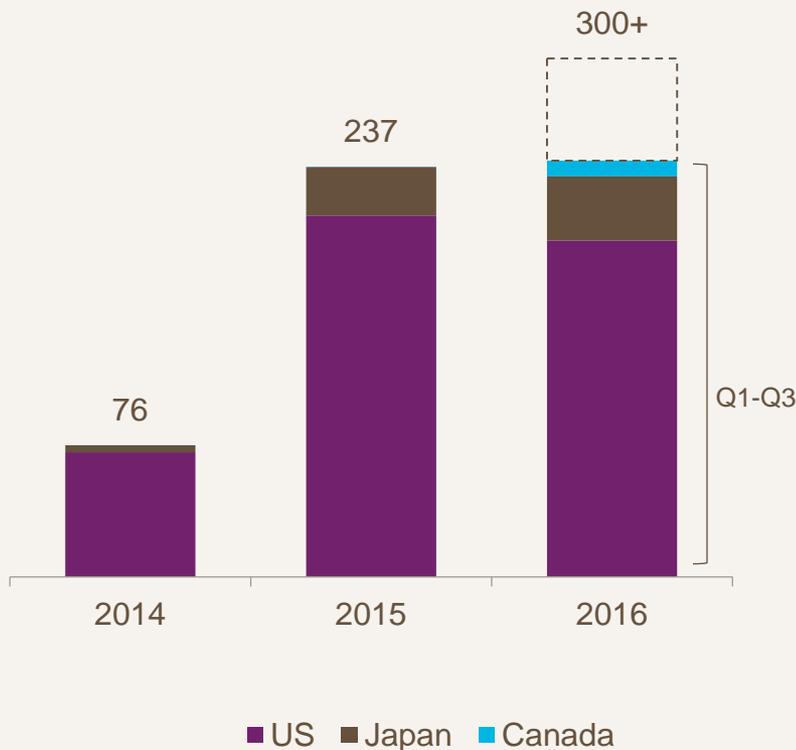
- Bypassing agents, ITI, potential bispecific antibody
- With few next generation therapies on the horizon, there is significant unmet medical need for therapies to treat inhibitor market



# ALPROLIX Has Also Delivered Strong Performance and Growth Opportunities Remain



## ALPROLIX Revenue (\$M)



- Approved for Hem B to reduce frequency of bleeding episodes with prophylactic infusions every 7 to 10 days, with potential to extend dosing based on individual response
- Advantage of more than two years of real-world experience and consistent long-term safety data

### Potential Growth Opportunities

- Continued shift to prophylaxis due to differentiated efficacy and dosing schedule, and shift to EHLs
- Expansion into new geographies
- Increased patient access
- Treating women who have the recessive gene for hemophilia

Source: Investor Presentation, Company Website, Form 10

# Evolution of the Hemophilia B Landscape

## Near Term

- Competition from other EHL entrants

## Longer Term

- Potential subcutaneous EHLs, including BIVV 002
- New MOAs including gene therapies, RNAi



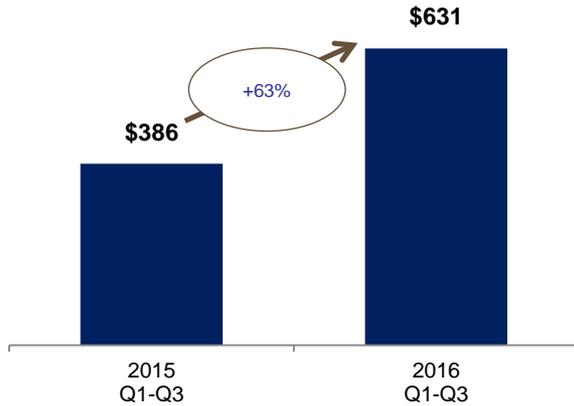


## Financial Overview

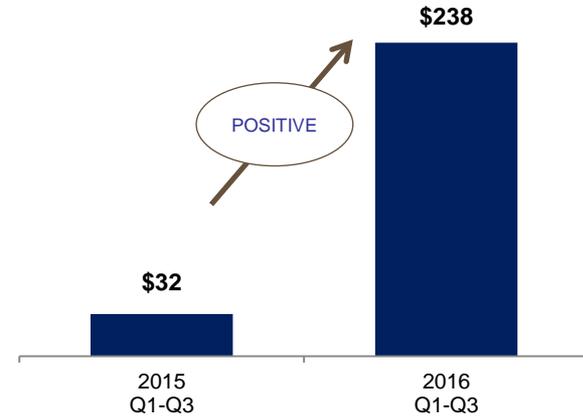
John Greene, CFO

# Financial Snapshot

## Revenues (\$M)



## Operating Income (\$M)



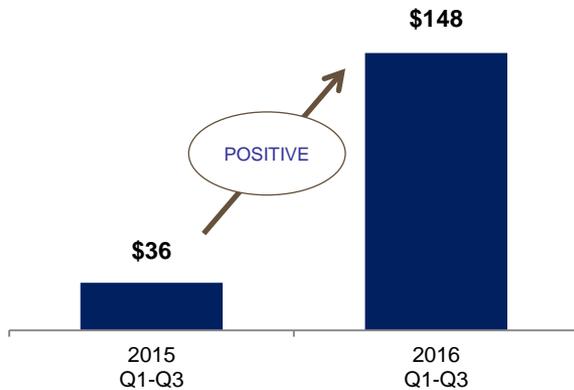
Gross Margin

2015 Q1-Q3: 87%  
2016 Q1-Q3: 79%

Operating Margin

2015 Q1-Q3: 8%  
2016 Q1-Q3: 38%

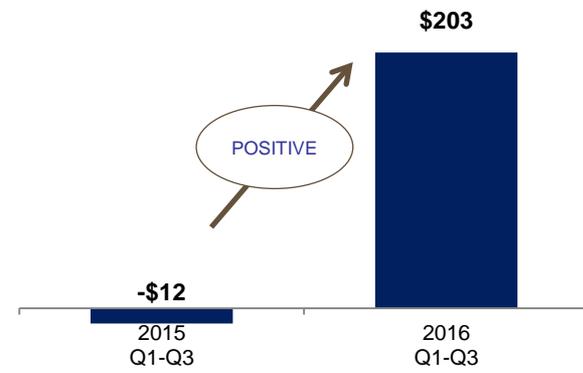
## Net Income (\$M)



Tax Rate

2015 Q1-Q3: 37.5%  
2016 Q1-Q3: 37.5%

## Free Cash Flows (\$M)



Cash as % of Net Income

2015 Q1-Q3: (33%)  
2016 Q1-Q3: 137%

Note: 2016 reported with Pro Forma adjustments

Free Cash Flows = Net CFs provided by (used in) operating activities + Net CFs used in investing activities

# 2017 Financial Guidance

## Guidance

## GAAP

## Non-GAAP

**Revenue  
Growth**

**17 – 19%**

**17 – 19%\***

**Operating  
Margin**

**38 – 42%**

**43 - 47%**

**Tax Rate**

**36 – 38%**

**36 – 38%\***

## Opening Balance Sheet

(In millions)

**Pro Forma  
as of Sept 30, 2016**

Total current assets \$575.5

Cash and cash equivalents \$325.0

Accounts receivable, net \$126.8

Inventory \$113.6

Other current assets \$10.1

Total current liabilities \$79.6

Net Working Capital \$495.9

Guidance as of January 6, 2017

Non-GAAP outlook excludes: One time separation and set up costs, equity based compensation and amortization of intangible assets. These items impact operating margin. The GAAP to Non-GAAP reconciliation of these items is included in the Appendix. Our guidance does not include the impact of potential business development.

\* No adjustment.



**Closing**

John Cox, CEO

# Our Commitment to the Hemophilia Community

## Humanitarian Aid Reaching Patients Worldwide



**178 M**  
IUs of clotting  
factor donated

**4,200**  
People treated  
in 38 countries

**4,600+**  
Acute bleeds

**300+**  
Surgeries,  
30 life-saving

Percentage of pediatric patients receiving treatment  
in these countries has nearly doubled  
(from 14% to 28%)



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# Appendix

# Income Statement

	2015 Full year	2015 Nine months end Sept 30	2016 Nine months end Sept 30	Adjust	2016 Pro Forma Nine months end Sept 30
Revenues:					
Product, net	554.1	381.7	604.8		604.8
Collaboration revenue	6.2	4.4	26.4		26.4
<b>Total revenues</b>	<b>560.3</b>	<b>386.1</b>	<b>631.2</b>		<b>631.2</b>
Cost and expenses:					
<b>Cost of sales</b>	<b>52.9</b>	<b>50.8</b>	<b>162.2</b>	(30.0) A	<b>132.2</b>
<i>Gross Margin %</i>	<i>91%</i>	<i>87%</i>			<i>79%</i>
Research and development	186.1	135.4	122.6		122.6
<i>% revenues</i>	<i>33%</i>	<i>35%</i>			<i>19%</i>
Selling, general and administrative	223.3	167.7	138.4		138.4
<i>% revenues</i>	<i>40%</i>	<i>43%</i>			<i>22%</i>
<b>Total operating expenses</b>	<b>409.4</b>	<b>303.1</b>	<b>261.0</b>		<b>261.0</b>
<b>Income from operations</b>	<b>98.0</b>	<b>32.2</b>	<b>208.0</b>	30.0	<b>238.0</b>
<b>Operating Margin %</b>	<b>17%</b>	<b>8%</b>			<b>38%</b>
Other income (expense), net	0.6	0.6	(1.0)		(1.0)
Income before income tax expense	98.6	32.8	207.0	30.0	237.0
Income tax (benefit) expense		(3.3)	(3.7)	3.7 B	0.0
	37.0			88.9 C	88.9
<b>Net income</b>	<b>61.6</b>	<b>36.1</b>	<b>210.7</b>	(62.6)	<b>148.1</b>

A - Elimination of accelerated depreciation associated with Bio 2

B - Reflects elimination of historical Bioverativ tax benefit

C - Reflects expected tax expense using an effective income tax rate of 37.5%

# GAAP/Non-GAAP reconciliation

## Operating margin reconciliation:

GAAP operating margin	38 - 42%
One time separation and set up costs	2%
Equity based compensation	2%
Amortization of intangibles	1%
Non-GAAP operating margin	43% - 47%

# Balance Sheet

	2015 Full year	2016 Nine months end Sept 30	Adjust	2016 Pro Forma Nine months end Sept 30
<b>Balance Sheet</b>				
<b>ASSETS</b>				
Current assets:				
Cash		0.0	325.0 A	325.0
Accounts receivable, net	94.4	126.8		126.8
Inventory	252.1	283.3	(169.7) B	113.6
Other current assets	4.0	10.3	(0.2) C	10.1
Total current assets	350.5	420.4	155.1	575.5
Property, plant and equipment, net	75.5	45.0	(26.9) C	18.1
Intangible assets, net	30.0	53.1		53.1
Other long-term assets	19.6	22.4		22.4
Total assets	475.6	540.9	128.2	669.1
<b>LIABILITIES AND EQUITY</b>				
Current liabilities:				
Accounts payable	10.8	12.3		12.3
Accrued expenses and other current liabilities	49.4	68.9	(1.6) C	67.3
Total current liabilities	60.2	81.2	(1.6)	79.6
Long-term liabilities	30.7	53.8		53.8
Total liabilities	90.9	135.0	(1.6)	133.4
Commitments and contingencies				
Equity:				
Net parent company investment	384.4	401.6	129.8	531.4
Accumulated other comprehensive loss	0.3	4.3		4.3
Total equity	384.7	405.9	129.8	535.7
Total liabilities and equity	475.6	540.9	128.2	669.1
<b>Working Capital</b>	<b>290.3</b>			<b>495.9</b>

A- Initial cash contribution from Biogen to Bioverativ

B - Drug substance (raw material and work-in progress inventory) retained by Biogen

C - Biogen manufacturing facility, related assets and liabilities that will not transfer to Bioverativ

# Sobi Collaboration

Royalty and Net Revenue Share Rates:	Method	Rates post Sobi Opt-In	
		Base Rate following 1st commercial sale in the Sobi Territory:	Rate during the Reimbursement Period:
Sobi rate to Biogen on net sales in the Sobi Territory	Royalty	12%	Base Rate plus 5%
Biogen rate to Sobi on net sales in the Biogen North America Territory	Royalty	12%	Base Rate less 5%
Biogen rate to Sobi on net sales in the Biogen Direct Territory	Royalty	17%	Base Rate less 5%
Biogen rate to Sobi on net revenue from the Biogen Distributor Territory	Net Revenue Share	50%	Base Rate less 15%

- For the years ended December 31, 2015, 2014 and 2013, the royalty payable to Sobi based upon sales in the company's territory was 2%. Upon Sobi's first commercial sale in 2016, and during the Reimbursement period, the royalty rate the company will pay Sobi on sales of ELOCTATE and ALPROLIX in our territory is 7%. After the Reimbursement period concludes, the royalty rate we pay to Sobi increases to 12%. We are recording cost of sales at the effective royalty rate expected over the term of the agreement of approximately 11%.
- The royalty rate received by the company, during the Reimbursement period on sales of ELOCTATE and ALPROLIX in Sobi's territory is 17%. After the Reimbursement period concludes, the royalty we receive decreases to 12%. We are recording revenue at the effective royalty rate expected over the term of the agreement of approximately 14%.

# Collaboration on San Raffaele's Lentiviral Platform for Hemophilia A and B

## Technology

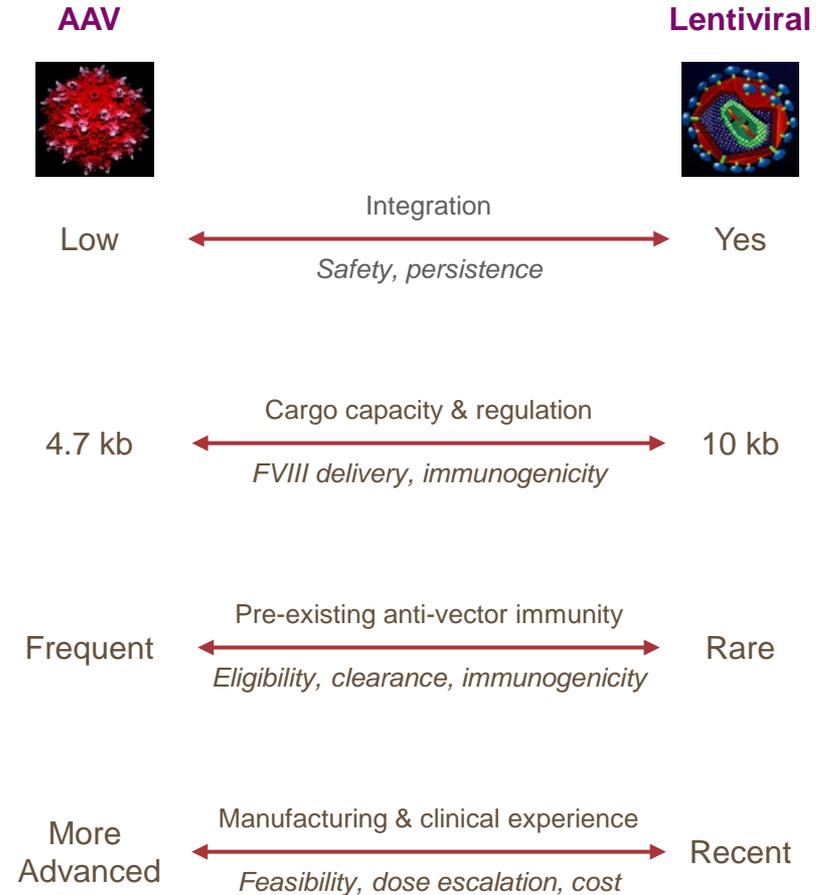
Leverages San Raffaele's expertise in lentiviral vector development and next-generation lentiviral platform

## Potential Clinical Profile

Potential to provide single-dose, lasting treatment for Hemophilia A and B patients

## Competitive Positioning

Persistent gene transfer in most tissues throughout development, addresses FVIII size challenge and may circumvent immunity limitations of AAV vectors utilized in majority of current gene therapy approaches. Lentivirus (self inactivated) Utilizes same 3rd generation Self-Inactivating Lentiviral technology used by the Naldini group to cure kids with WAS and MLD by ex vivo treatment of hematopoietic stem cells, without any signs of insertional oncogenesis



# Leading Medical Experts in Sickle Cell Disease and a Portfolio of Research Stage Assets

## Technology

Small molecule approaches primarily addressing the causative defect leading to the pathophysiology of SCD

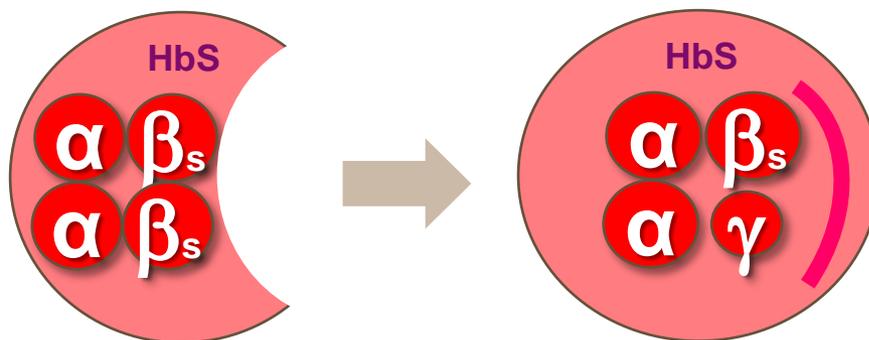
## Potential Clinical Profile

Goal is to develop disease-modifying therapies to treat significant unmet needs in SCD

## Competitive Positioning

Opportunity to nurture a robust discovery pipeline that could make BIVV the only company with a comprehensive approach to addressing SCD

Fragmented competition



**Diseased**

- Sickling
- Hemolytic anemia
- Sickle crises

**Healthy**