## Swedish Orphan Biovitrum

Jefferies Global Healthcare Conference

New York, 5 June 2012







Geoffrey McDonough

CEO



#### Safe Harbor Disclaimer

In order to utilize the 'Safe Harbor' provisions of the United States Private Securities Litigation Reform Act of 1995, Swedish Orphan Biovitrum is providing the following cautionary statement. This presentation contains forward-looking statements with respect to the financial condition, results of operations and businesses of Swedish Orphan Biovitrum. By their nature, forward-looking statements and forecasts involve risk and uncertainty because they relate to events and depend on circumstances that will occur in the future. There are a number of factors that could cause actual results and developments to differ materially from that expressed or implied by these forward-looking statements. These factors include, among other things, the loss or expiration of patents, marketing exclusivity or trade marks; exchange rate fluctuations; the risk that R&D will not yield new products that achieve commercial success; the impact of competition, price controls and price reductions; taxation risks; the risk of substantial product liability claims; the impact of any failure by third parties to supply materials or services; the risk of delay to new product launches; the difficulties of obtaining and maintaining governmental approvals for products; the risk of failure to observe ongoing regulatory oversight; the risk that new products do not perform as we expect; and the risk of environmental liabilities.



#### Who We Are

Sobi is an innovative commercial stage bio-pharmaceutical company with a leading position in niche and rare disease therapies.



Our operations are driven by a diversified, profitable, growth-oriented product portfolio.



We have a **late-stage pipeline** with substantial commercial potential.



We have **world-class capabilities** in protein biochemistry and biologics manufacturing development – **validated by leading industry partners**.



## **Quick Facts**

• Listing: NASDAQ OMX (STO:SOBI)

- Outstanding shares: 265.2 M

- Sponsored ADR through BNY Mellon (SWTUY)

Market Cap: \$835 M

- Share price, close 1 June 2012: SEK 22.8

- 52-week range: SEK 11.40 – 26.50

#### Ownership Summary:

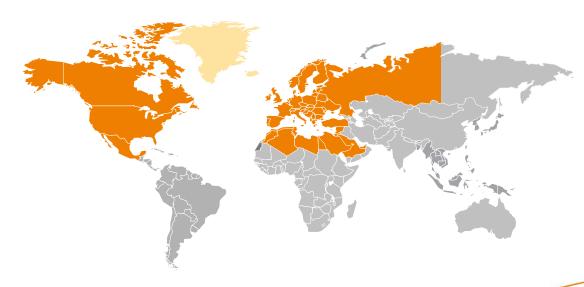
31 March 2012	% of capital
Sweden	63
of which Investor AB	40
Foreign	37

• **Net Revenues 2011:** \$265 M

• **EBITA**<sup>1</sup> **2011:** \$17.6 M

• Net Debt, March 31, 2012: \$24.7 M

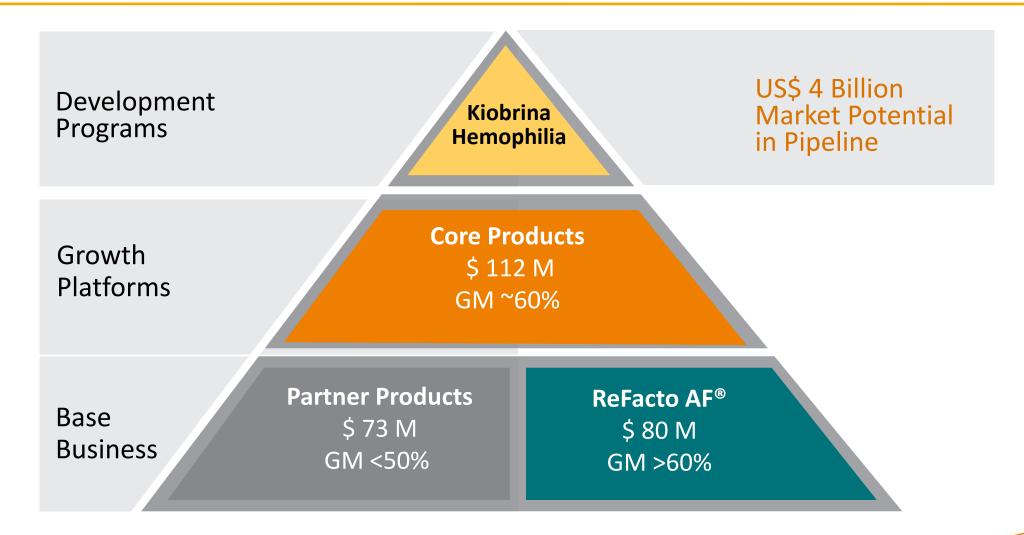
• **Employees:** ~ 500



<sup>&</sup>lt;sup>1</sup> Before non-recurring items. Figures restated in US dollars @ SEK 7.2234/dollar



## Revenues by Business Lines 2011



<sup>\*</sup>Figures restated in US dollars @ SEK 7.2234/dollar



## **Current Operating Business**

#### **Growth Platform**

#### **Core Products**

#### **Inflammation**

- Kineret
- Ruconest

#### **Genetics + Metabolism**

- Orfadin
- Ammonaps
- Ammonul

#### **Base Business**

#### **Partner Products**

#### **Specialty Indications**

- Kepivance
- Yondelis
- Ferriprox
- Betapred
- Buronil
- Aloxi
- Willfact
- > 40 products (each with annual sales < SEK 15 M)</li>

#### ReFacto AF®

- Manufacturing
- Royalty



#### Core Products - Kineret®

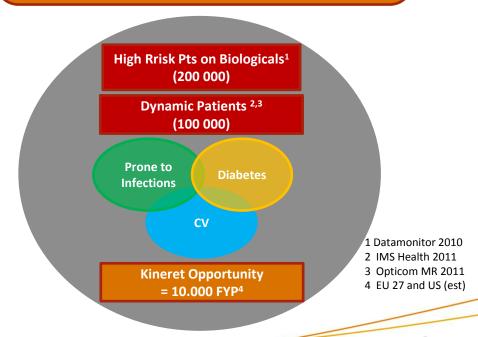




4 quarters rolling using average exchange rates for 2009 as base. Figures restated in US dollars @ SEK 7.2234/dollar

- Biological inhibitor of IL-1 Receptor
  - Unique mechanism of action

High risk RA-patients with co-morbidities for which the short half life and safety profile of Kineret are uniquely suitable

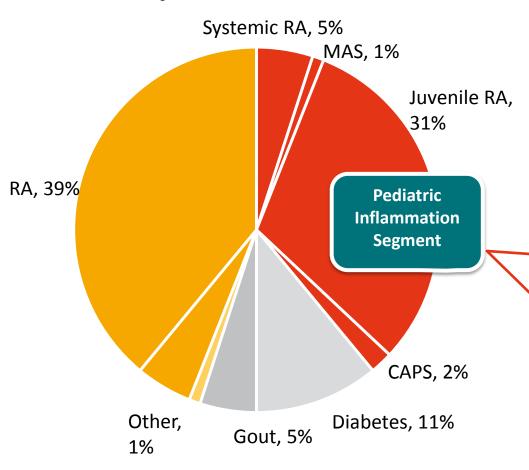




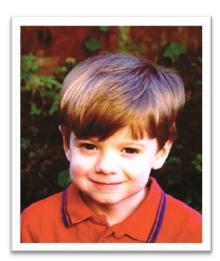
## Positioning Kineret in Rare Disease Segment



#### **Prescriptions for Kineret®**







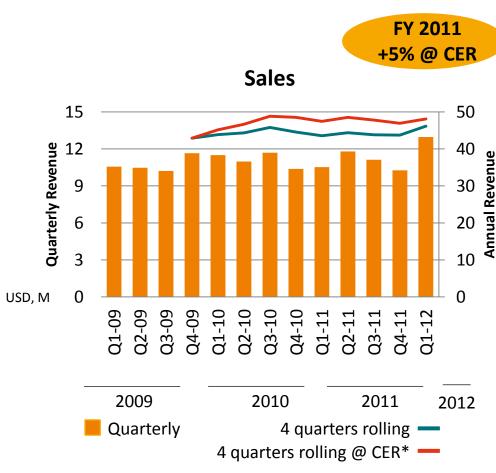
- Sobi will file for NOMID in US and CAPS in EU in 2012
- Critical to allow us to support practice in children

Source: Wolters Kluwer Claims Database, (April 2010 – July 2011)



### Core Products - Orfadin®





4 quarters rolling using average exchange rates for 2009 as base. Figures restated in US dollars @ SEK 7.2234/dollar

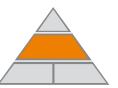
- Used for treatment of hereditary tyrosinemia type 1 (HT-1)
  - Tyrosineand toxic metabolites accumulate
  - Affects about one child in 100,000



- Increasingly included in new born screening programs
  - -US and Europe
- Growth in Russia, Eastern Europe, South America and Middle East



### Orfadin® - New Formulation Meets Market Need



#### Rationale

- 1. Enables precise dosing
- 2. Expands compliance
- 3. IP Extension (orphan status EU 2017)

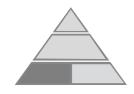
#### **Status**

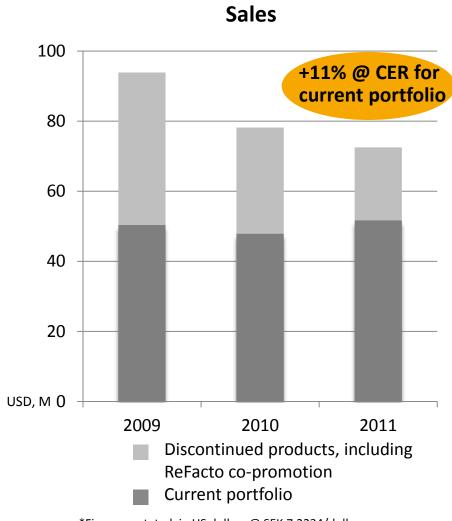
- Manufacturing scale-up underway
- Clinical program underway
- EMA PIP approved March 2012
- FDA discussion with FDA in 2012





#### **Partner Products**





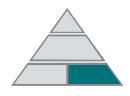
## Offers small/midsized companies an integrated solution for commercialization of products

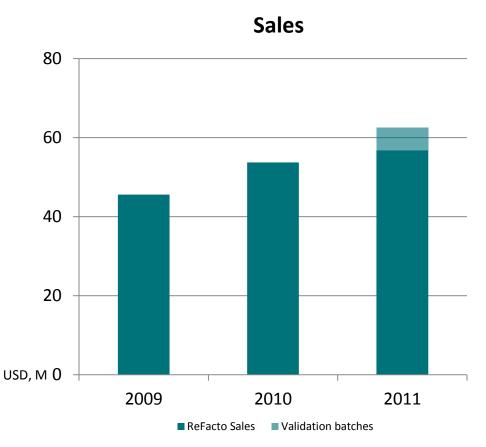
- Market leader in Nordics, Baltics
- Expanding to CEE and Russia
- About 40 specialty pharmaceuticals
- Key therapeutic areas are hematology, oncology and emergency medicines
- Strong underlying growth in 2011 and Q1 2012
- Co-promotion rights returned to Pfizer in Feb 2012 for payment of \$47.5 M



<sup>\*</sup>Figures restated in US dollars @ SEK 7.2234/dollar

### ReFacto – A Durable Platform



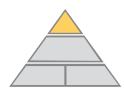


ReFacto includes manufacturing and royalty revenues Figures restated in US dollars @ SEK 7.2234/dollar

- Long-standing exclusive partnership with Pfizer
  - Global manufacturer of drug substance for ReFacto AF/ Xyntha® (hemophilia A)
- Revenues from product manufacturing and royalty on Pfizer's global sales
  - Pfizer expanding in emerging markets
- Supply agreement extended to 2020, with option to renew



## Our Pipeline



Indication	Product/Project	Partner	Phase I	Phase II	Phase III	Reg Phase
Hemophilia A	rFVIIIFc	biogen idec				
Hemophilia B	rFIXFc	biogen idec				
Prevent growth restric- tion in preterm infants	Kiobrina					
Diuresis and seizures in neonates	Bumetanide (reformulated)	Only For Children Pharmaceuticals Innovating For Child Care				

### Life Cycle Management

Indication	Product/Project
CAPS	Kineret <sup>®</sup>
Hereditary tyrosinemia type 1	Orfadin <sup>®</sup> , liquid formulation



## Long Acting Recombinant Factor 9 and Factor 8

Life long therapy for Hemophilia B (Factor 9) and A (Factor 8)

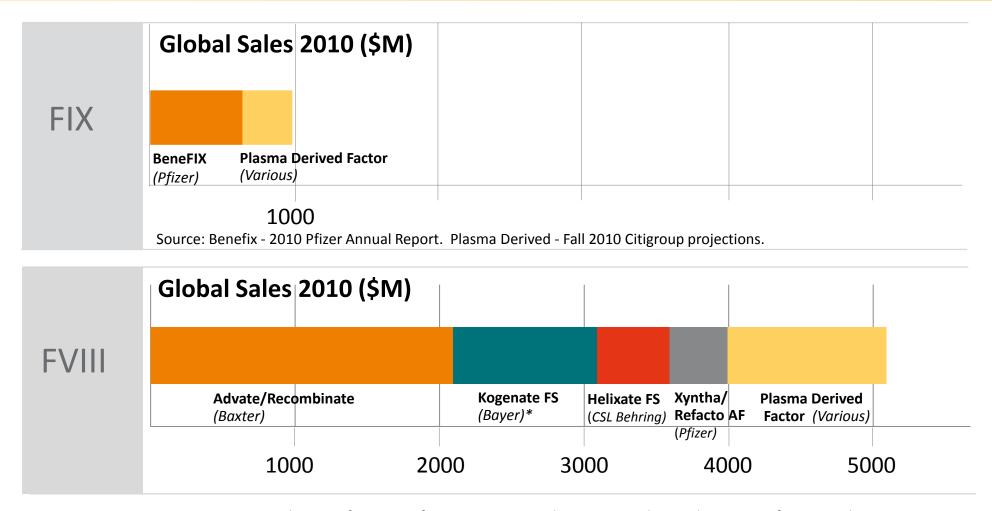




- 1. Programs run by Biogen Idec
- Pivotal data due H2 2012
- 3. Sobi and Biogen share economics through cross-royalty structure
- 4. Sobi has rights in Europe, Russia, Middle East, and North Africa



## Hemophilia B and A Represent a Combined \$6+ Billion Market With Tremendous Potential for Differentiated Products



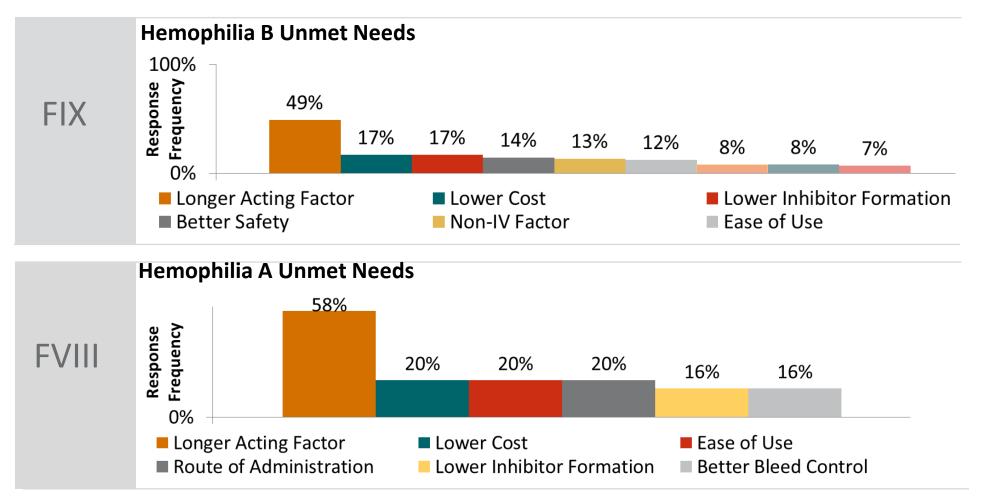
Source: 2010 Annual reports for Bayer, Pfizer, Baxter. Internal estimates and secondary reports for remainder.





<sup>\*</sup>Excludes sales to CSL Behring

## Longer Lasting Agents Most Desired Improvement

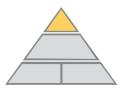


Source: Market research. N=92





## Hemophilia is a \$3.4B Market in Sobi Territory



#### FIX

#### Hemophilia B

- Europe \$380M
  - 44% of global sales
  - ~ 4,000 patients

#### **FVIII**

#### Hemophilia A

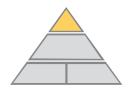
- Europe \$3B
  - 55% of global sales
  - ~ 22,000 patients



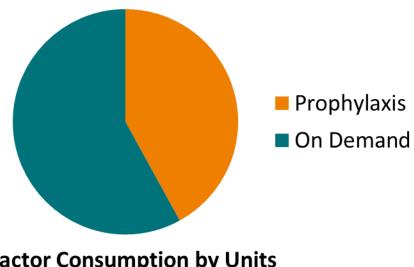




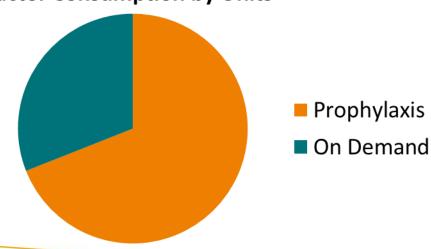




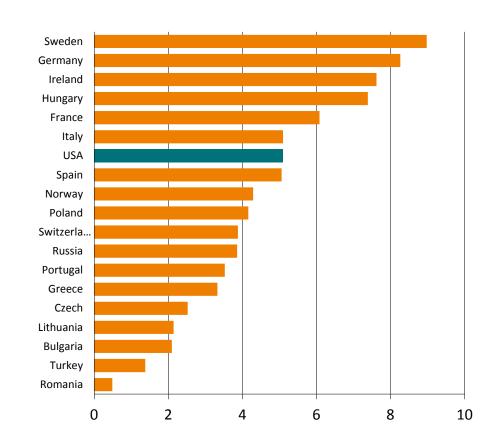
#### **Factor Consumption by Patients**



#### **Factor Consumption by Units**



#### Per Capita consumption of FVIII (IU/Inhab.)

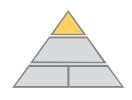


Source: WFH 2009

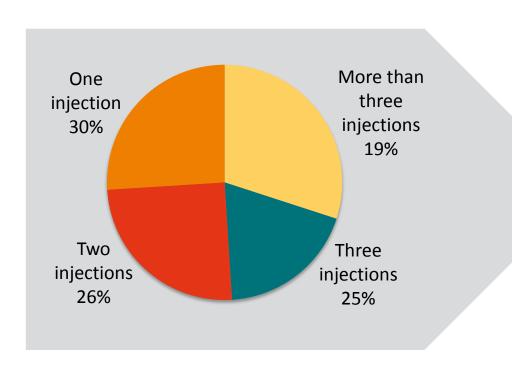




# Significant Opportunity to Improve On-Demand Care



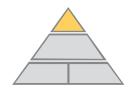
No. of injections given when bleedings occur.

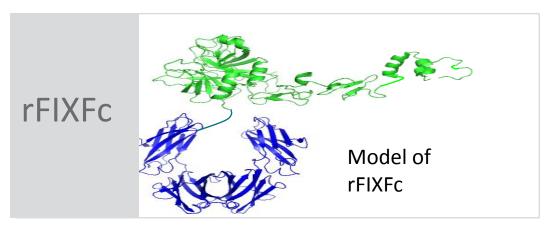


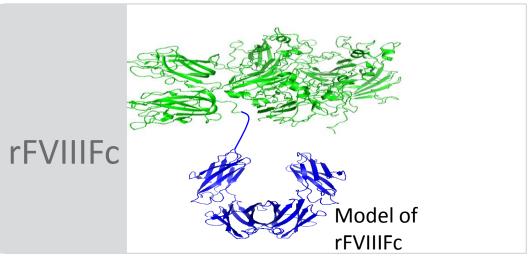
70% of EU patients
treated on-demand
use
more than
1 injection per bleed

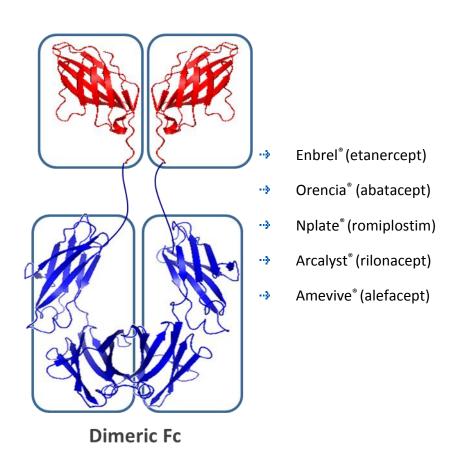


## Fc Fusion Technology Well Understood







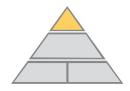


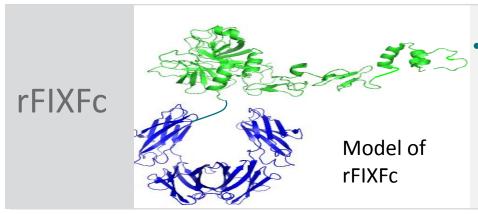
1Summary of product characteristics of BeneFIX® (Nov 18, 2009)
2 Blood 2011 blood-2011-07-367003; published ahead of print November 22, 2011, doi:10.1182/blood-2011-07-367003
3 15/16 PTPs were included for PK analysis (1 subject did not complete PK profiling); In press, Blood 2011





## Phase II Studies Confirm Long Half-Life

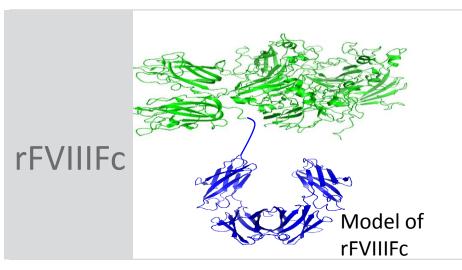




~3 x increase in half-life relative to historical data for BeneFIX®

PK parameter Elimination  $T_{\chi}$  (hours)<sup>2</sup>

rFIXFc n=11 mean 57 h (42–75 h) BeneFIX<sup>®1</sup> n=56 mean 19 h (11–36 h)



- ~1,5-1,75 fold increase in half-life versus Advate®
- All patients had a longer half life

PK parameter Elimination  $T_{1/2}$  (hours)<sup>3</sup>

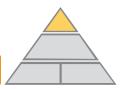
rFVIIIFc n=15 mean 19 h (18-22 h) Advate® n=15 mean 11,5 h (10-13h

1Summary of product characteristics of BeneFIX® (Nov 18, 2009)
2 Blood 2011 blood-2011-07-367003; published ahead of print November 22, 2011, doi:10.1182/blood-2011-07-367003
3 15/16 PTPs were included for PK analysis (1 subject did not complete PK profiling); In press, Blood 2011





## Pivotal Studies To Assess Prophylaxis + On Demand



#### **B-LONG**

#### rFIXFc

• An Open-Label, Multicenter study

 Evaluation of Safety, Pharmacokinetics and Efficacy in the Prevention and Treatment of Bleeding in Previously Treated Subjects With Severe Hemophilia B

Arm 1 Low Dose Prophylaxis

High Dose Prophylaxis

Arm 2

High Dose Prophylaxis
High Dose Regimen (A-LONG)

Screening

#### **A-LONG**



An Open-Label, Multicenter Study

 Evaluation of Safety, Pharmacokinetics and Efficacy in the Prevention and Treatment of Bleeding in Previously Treated Subjects With Severe Hemophilia A Arm 3 On-demand Regimen

Arm 4

Surgery (B-LONG only)

B-LONG has 4 different treatment groups and A-LONG has 3.

More information, please visit:

www.clinicaltrials.gov www.biogenidechemophilia.com



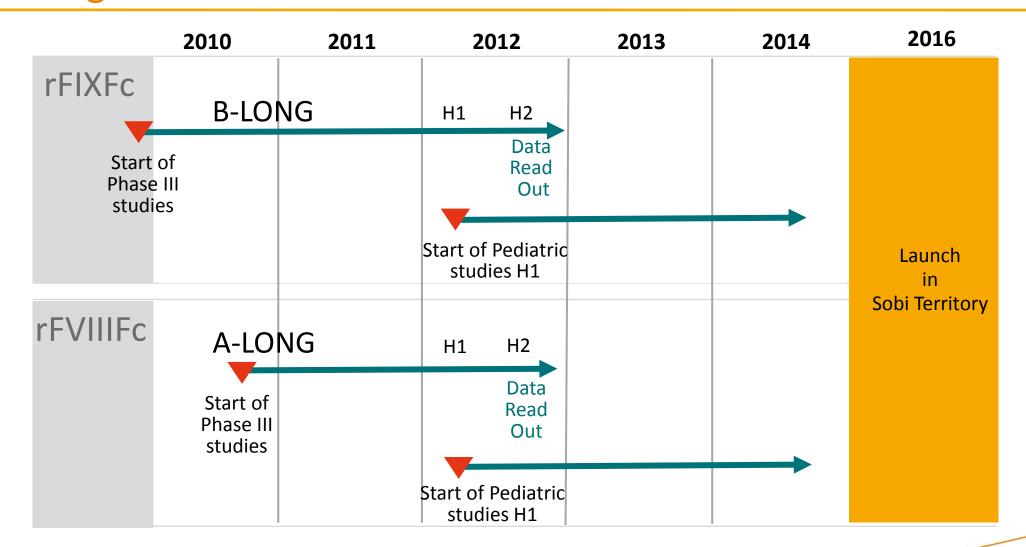


## S

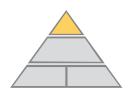


biogen idec

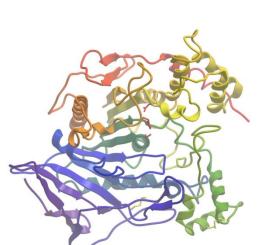
## Program Timelines: Sobi Territories Follow US



# Kiobrina – Oral Enzyme Replacement for Premature Infants



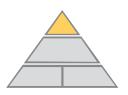




- Kiobrina is recombinant human Bile Salt Stimulated Lipase (rhBSSL) given together with formula or pasteurized milk to restore BSSL activity in the infant GI tract and to support growth in the neonatal period
- BSSL needed in the infant GI tract to digest long chain fatty acids
- LCA essential for neonatal growth and development
- 3. Premature infants do not produce BSSL
  - a. BSSL naturally occurs in mother's milk
  - b. BSSL not present in pasteurized milk or formula
  - Lack of BSSL is correlated with impaired growth in neonatal period



# Aim to Improve Growth Reduce NICU Costs + Improve Development Outcomes



- Earlier discharge
- Reduced Hospital Utilization
- Reduced Illness due to prolonged hospitalization



Cost saved if discharged 1 week earlier from NICU

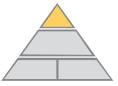


- Improved functional outcomes
- Reduced risk of neurodevelopment delay

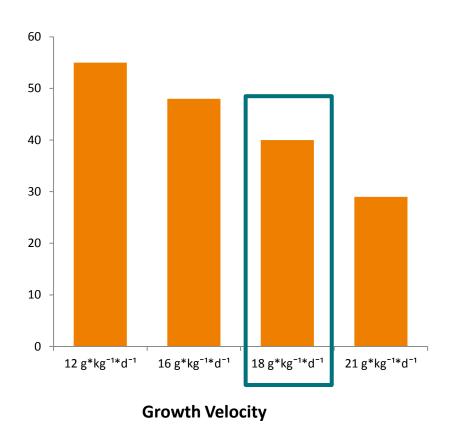
Improved developmental and functional outcomes in childhood



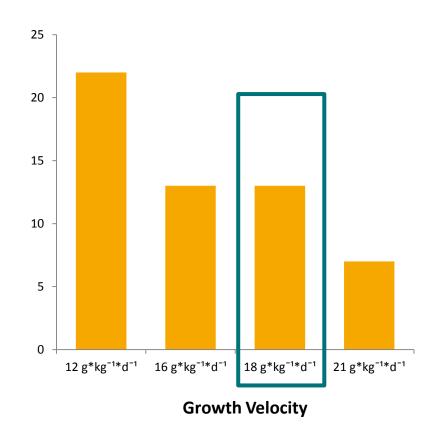




#### % Patients w/ Impaired Neurodevelopment



% Patients w/ Cerebral Palsy

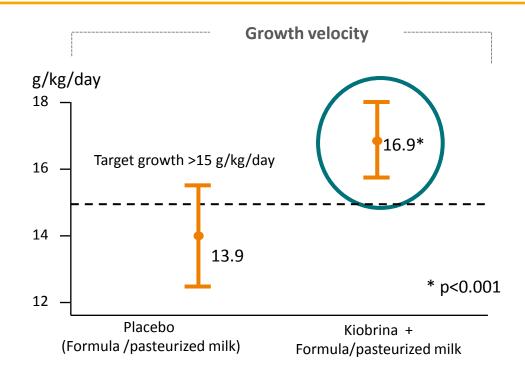


Ehrenkranz et al. Pediatrics, 2006; 117;1253 Previously premature infants assessed at 18 – 22 mo of age, n=494.



## Phase II: Potentially Significant Medical Value

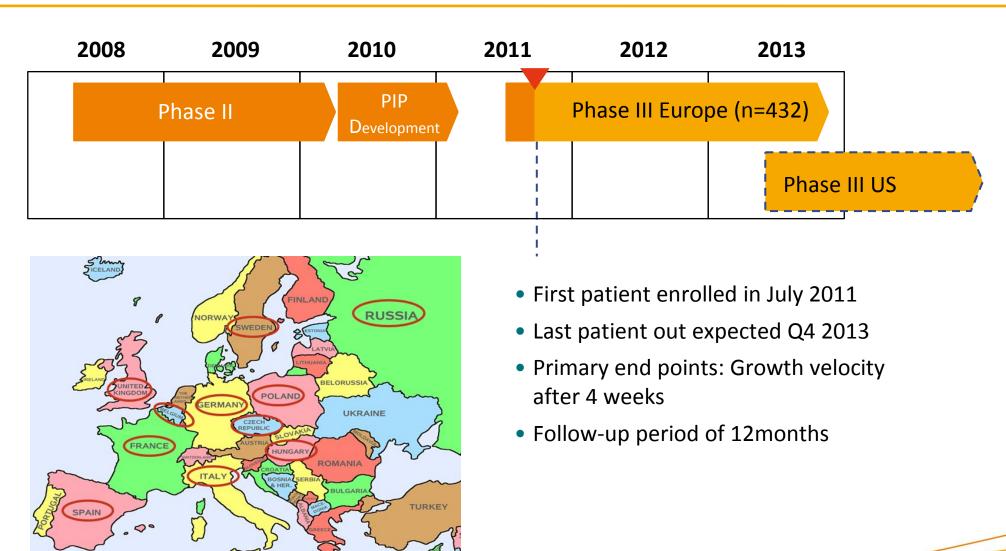
- Phase II data show Kiobrina accelerated growth after one week of treatment
- Ongoing Phase III study uses same primary endpoint of Growth Velocity, with four weeks treatment and 12 months observation period



The phase II Kiobrina program was designed as two parallel prospective randomized double-blind crossover studies where Kiobrina, or placebo, was administered in pasteurized milk, or preterm infant formula, during one week of treatment. All infants were born before week 32 of gestational age.

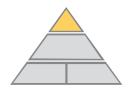


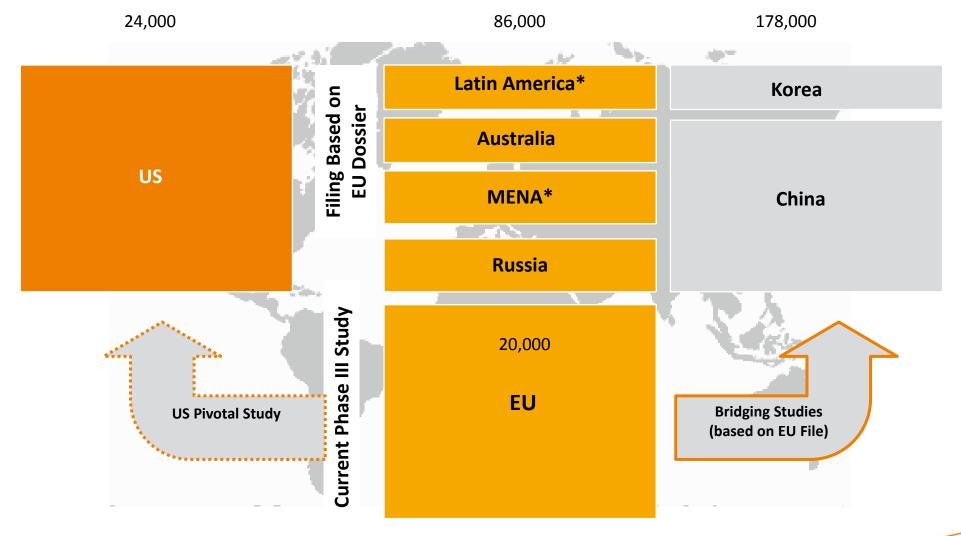
## SPA-Approved Pivotal Trial Underway in Europe





## Kiobrina Could Help >275,000 Patients Globally\*





<sup>\*</sup>Lat AM: Brazil, Mexico, Argentina, Venezuela



<sup>\*</sup>MENA: Saudi, Iran, Israel, Egypt, Turkey

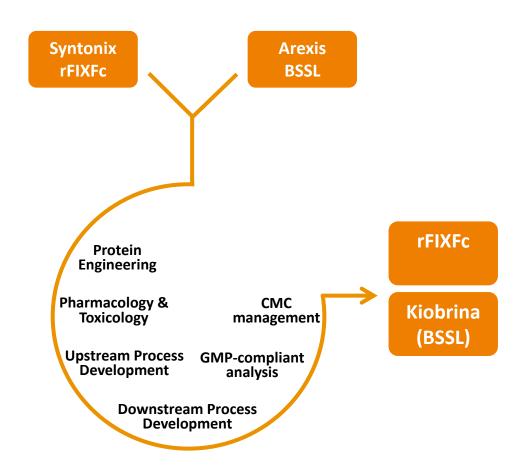
## Leveraging Strategic Biologics Development Capabilities

## Value creation for innovative biologics therapies through:

- Protein biochemistry capabilities in late preclinical development
- GMP biologics process development and

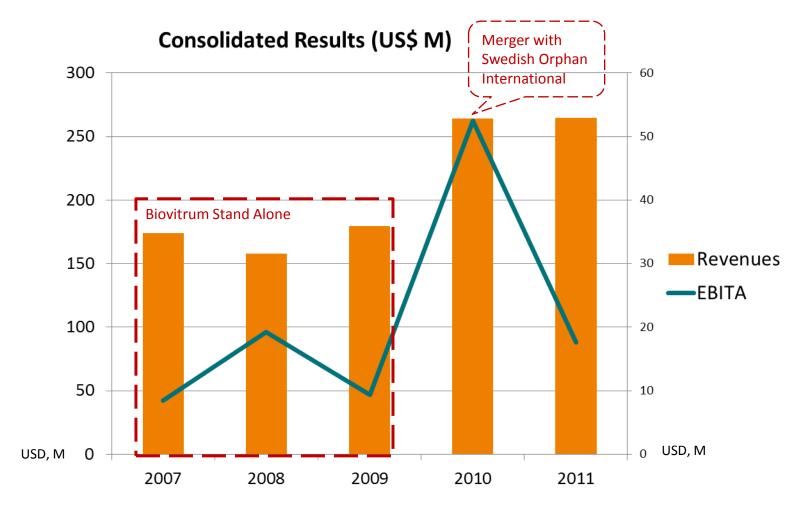
#### **Innovation Model Relies on Partnerships:**

- →Inbound to Biologicals Development
- →Outbound for Clinical Development





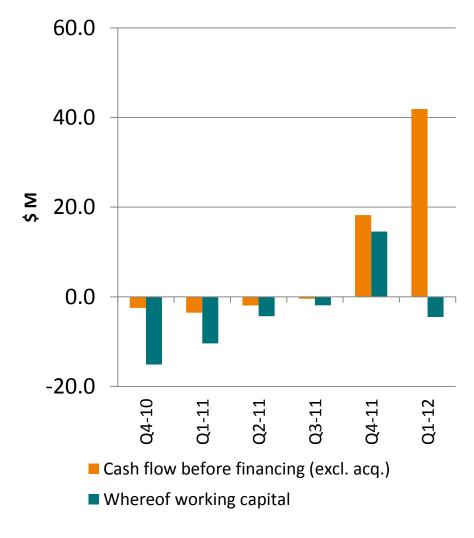
## Focus on Revenue Growth + Profitability

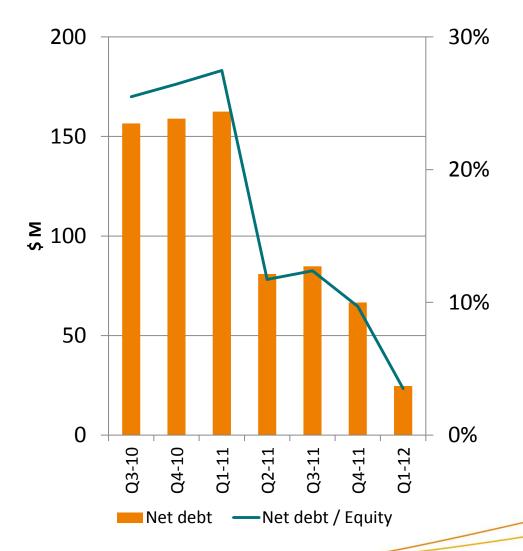


Figures restated in US dollars @ SEK 7.2234/dollar EBITA 2011 includes write-down of ~\$30M



### Cash Flow + Net Debt





Figures restated in US dollars @ SEK 7.2234/dollar



## 2012 Calendar Highlights

Event	H1 2012	H2 2012
Orfadin Liquid Formulation PIP Response		
Kineret CAPS PIP Response		
Complete Tech Transfer Kineret Manufacturing		
Kineret NOMID Filing FDA		
Kineret CAPS Filing EMA		
Top-Line Data for rFVIIIFc + rFIXFc Programs (BIIB)		
Kiobrina Complete Phase 3 Enrollment		

## Summary

Sobi is a commercial stage bio-pharmaceutical company with a leading position in rare diseases.

- Diversified, profitable, growth-oriented product portfolio.
- **2. Late-stage pipeline** with substantial commercial potential.



**Pioneer & Partner in Rare Diseases** 

3. World-class capabilities in protein biochemistry and biologics manufacturing development – validated by leading industry partners.

