# Swedish Orphan Biovitrum AB

## Annual General Meeting 2014

Geoffrey McDonough, CEO and President



Stockholm | 8 May 2014

sobi
Pioneer in Rare Diseases

# Sobi is an international specialty healthcare company dedicated to rare diseases

Our key therapeutic areas are Inflammation and Genetic diseases, with a growing focus on Haemophilia.

We deliver products to specialist physicians and their patients through our integrated and focused team approach to sales and marketing, medical affairs and patient access.

We leverage our world-class capabilities in protein biochemistry and biologics manufacturing to develop next generation biological products.

# Quick Facts Today

**Market Cap:** $3.2 Billion USD (SEK 20.8Billion)

**Share:** Close May 2, 2014: SEK 76.75
52-week range: SEK 36.80 – 87.20

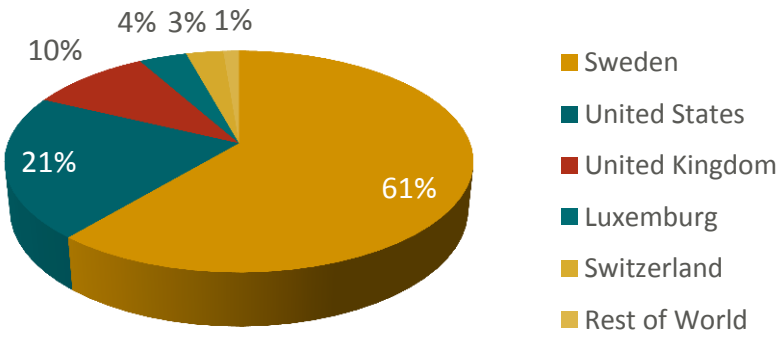
**Listing:** NASDAQ OMX (STO:SOBI)

Outstanding shares: 270.4 M

**International Presence**

- 550 employees
- Sales and marketing organization which covers about 20 countries in Europe
- Growing organisations in US, Russia, Middle East

**Ownership Summary:**
28 February 2014




*US$ 1 = SEK 6.53 (Exchange rate 2 May 2014)*

# 2013 Highlights

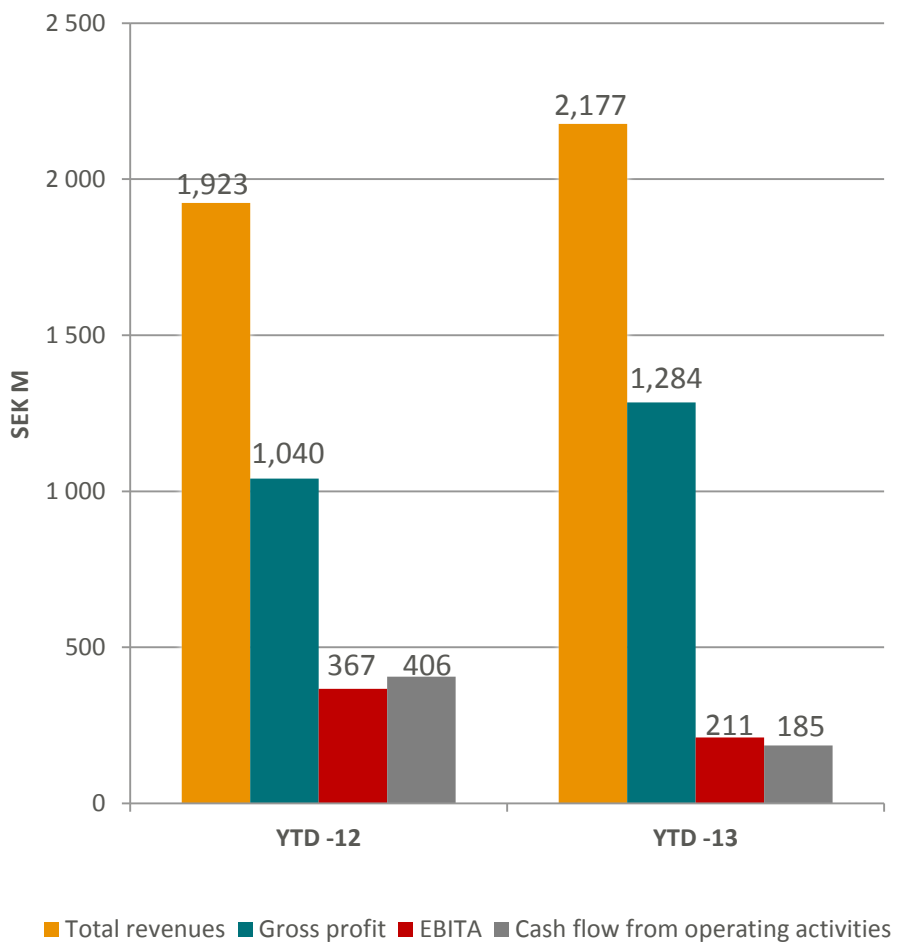
## Commercial

- Acquired the full commercial rights for Kineret® from Amgen
- Signed partnership agreements with:
  - Auxilium Pharmaceuticals for the development and commercialisation of Xiapex®
  - PharmaSwiss for marketing of Megace®, Monopril®, Cefzil® and Duricef®
  - Exelixis to support the distribution and commercialisation of Cometriq®
  - Gained rights to distribute Ravicti® in Middle East from Hyperion Therapeutics, Inc.
- Moved to Nasdaq OMX Large Cap

## Development

- Announced novel Complement C5 inhibitor program to enter phase 1 (SOBI002)
- Acquired additional phase 3 data for Kepivance®
- Submitted application for Orfadin oral suspension to EMA
- Received approval for Kineret for treatment of rare disease CAPS in the EU
- Phase 3 data for haemophilia programs confirmed long-lasting characteristics of Eloctate™ and Alprolix™ in both adults and children

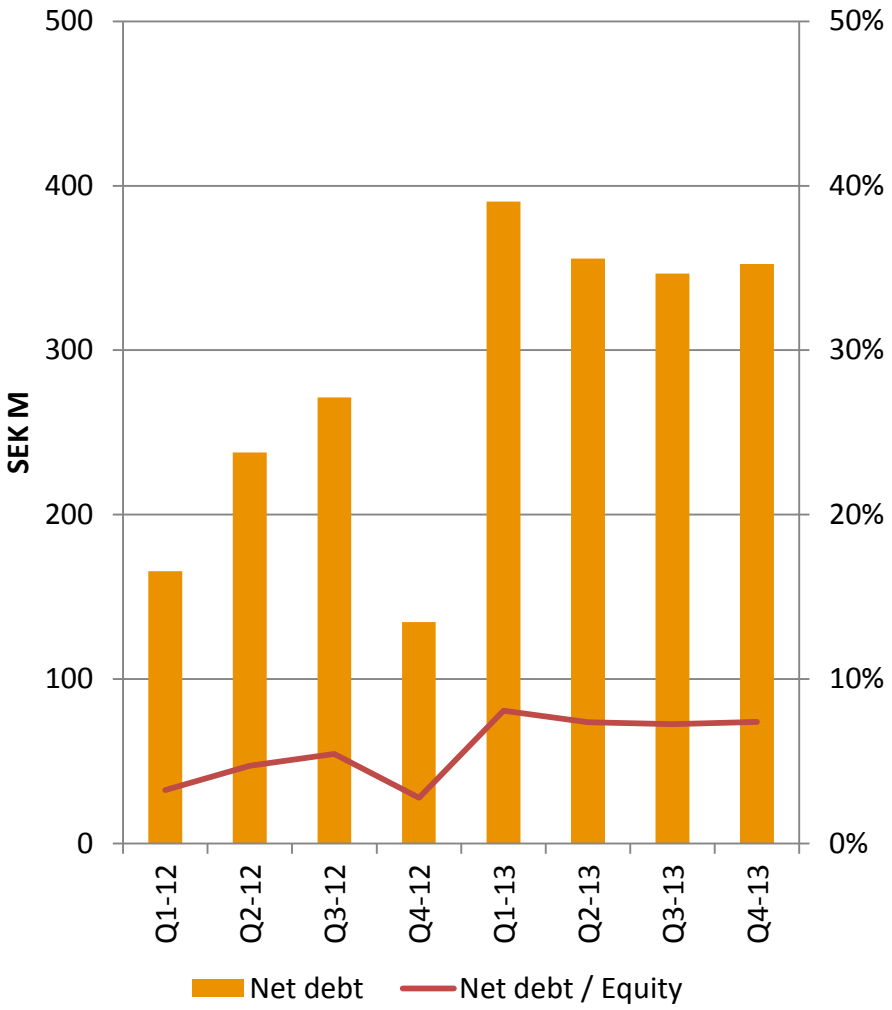
# Consolidated Results Full Year 2013




## Financial Highlights

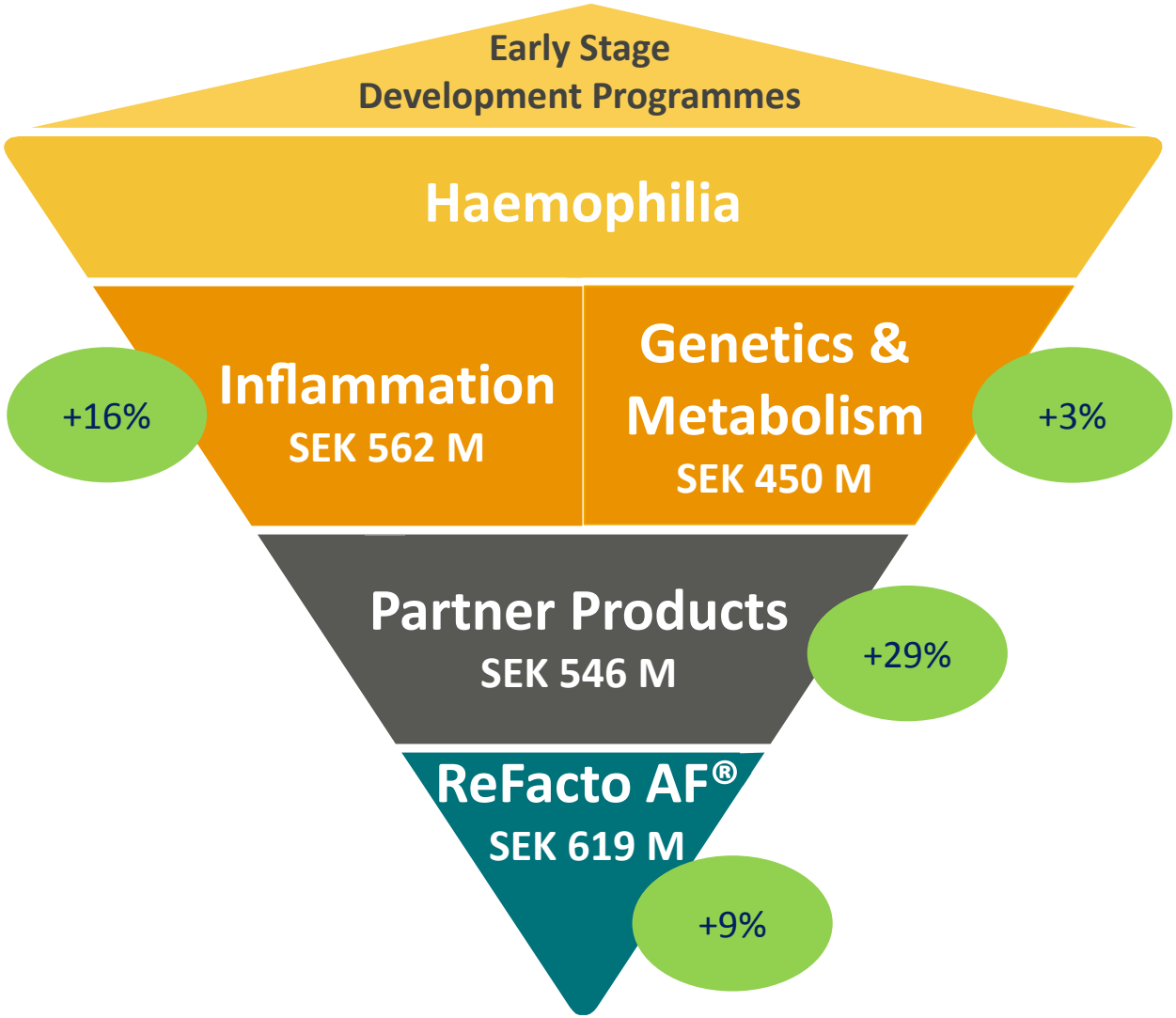
- Total revenues: SEK 2,177 M (1,923)
  - an increase of 13%
- Product revenues: SEK 1,558 M (1,344)
  - an increase of 16%
- Gross Margin: 59% (54)
- EBITA: SEK 211 M (367)
- Cash Flow from operations: SEK 185 M (406)
- Year-end cash position: SEK 445 M

# Net Debt




- Operating business is cash flow positive
- Cash Position SEK 445 M

# A Diverse, Growing Business Platform




Medium term investments to support commercialisation of the late stage Pipeline

Continued focus on growth in key Therapeutic Areas

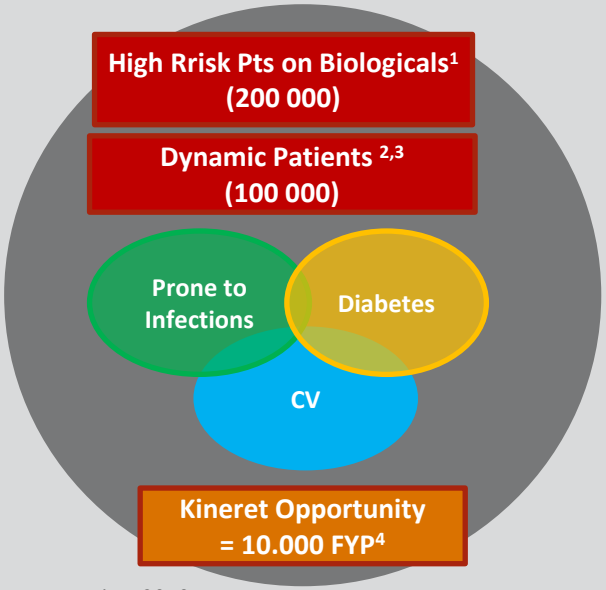
An integrated solution for commercialisation of niche and specialty products

Stable contribution to revenues from drug production and royalties

# Evolving Position of Kineret in IL-1 Diseases

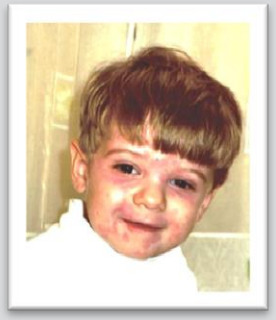
## RA Patients with Co-morbidity

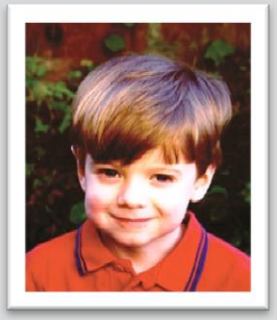
- Where Kineret's short half-life + safety profile uniquely suitable




1 Datamonitor 2010
2 IMS Health 2011
3 Opticom MR 2011
4 EU 27 and US (est)

## Pediatric Inflammation





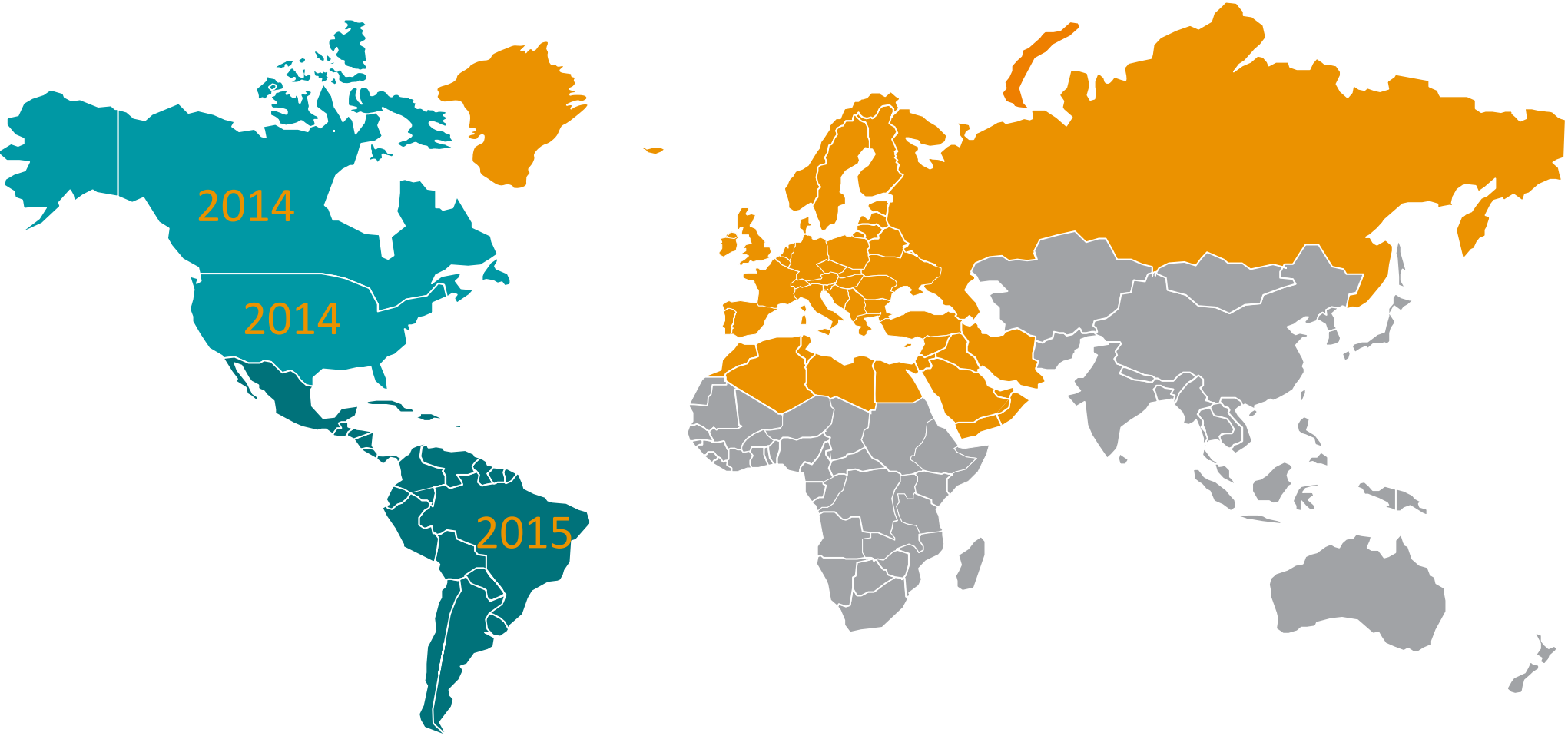
- NOMID approved in US YE 2012
- CAPS approved in EU in 2013

## Growing interest for role of IL-1

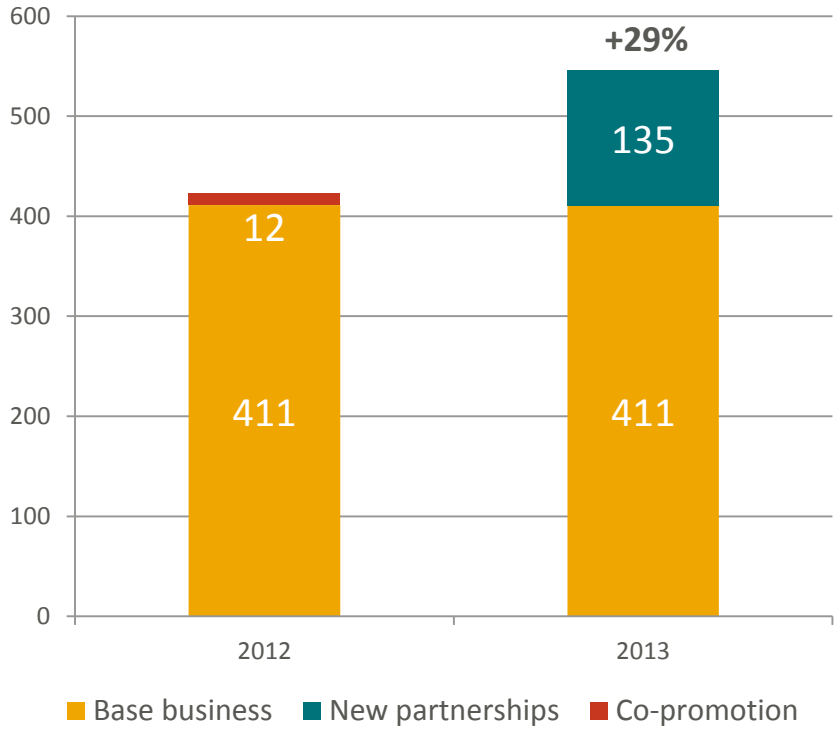
- Gout
- Post-myocardial infarction heart failure
- Stroke
- Skin disease (hidradenitis suppurativa, acne)
- Type 1 & 2 diabetes
- Dry eye syndrome
- Malignancies

Treating inflammation by blocking interleukin-1 in a broad spectrum of diseases
*Charles A. Dinarello, Anna Simon and Jos W. M. van der Meer, Nature Reviews, Aug, 2012*

# Taking Orfadin Direct in the Americas



# Partner Products: Strong Partnerships + Platform


** The Base business numbers includes discontinued products of SEK 27 M for 2012 and SEK 6 M for 2013. Excluding these, the base business grew 5 per cent.*

# Two Years Ago

## Actions to Reach Our Goals

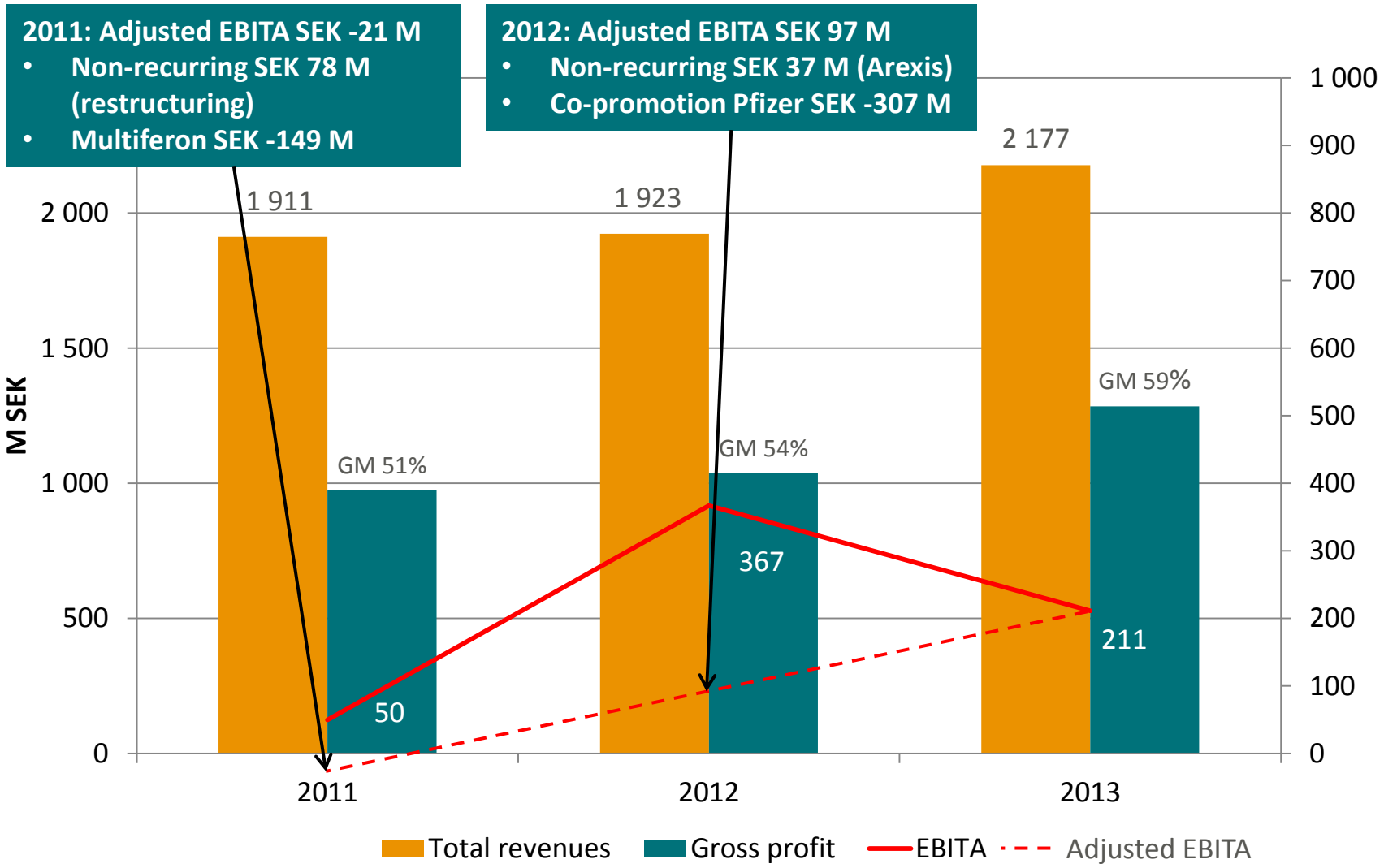
21.60 SEK

We intend to earn our way into our future based on **operational performance.**

1. Revenue growth through focus on key products
2. Ongoing cost discipline
3. Gross Margin improvement

➔ Improving cash flow from operations and working capital

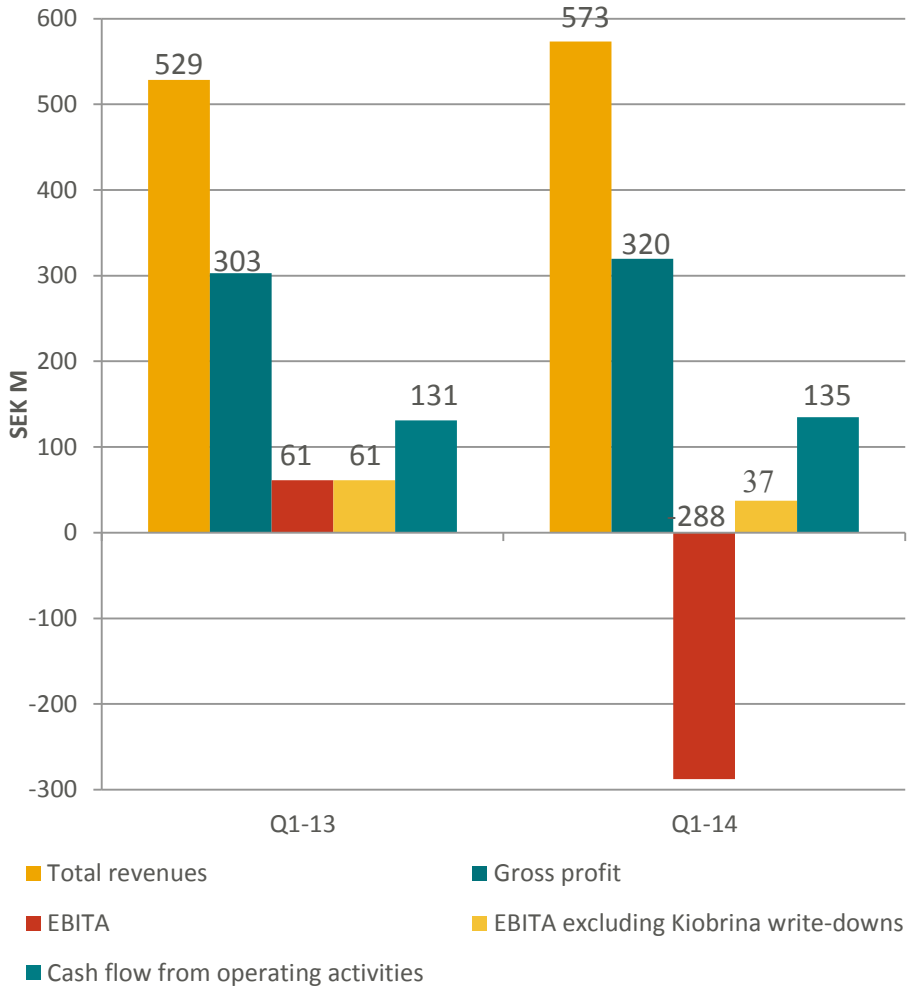
# Results in Perspective: 2011 – 2013



# Q1 2014 Financial Summary

**Financial Q1 2014 (Q1 2013)**

- Total revenues: SEK 573 M (529)
  - An increase of 8%
- Product revenues: SEK 406 M (345)
  - An increase of 18%
- Gross Margin: 56% (57)
- EBITA: SEK -288 M (61)
- EBITA excluding Kiobrina write-off SEK 37 M
- Cash flow from operating activities: SEK 135 M (131)
- End of quarter cash position: SEK 574 M



# Two Years of Progress

## 76.75 SEK

# Outlook 2014

## Revenues

MSEK 2,300 to 2,500

## Gross Margin

58-60%

## Operating costs

Operating costs are expected to increase as the company continues to prepare for the planned launch of the Haemophilia programmes.

*The outlook was first published in the 2013 Q4 report on 20 February 2014.*

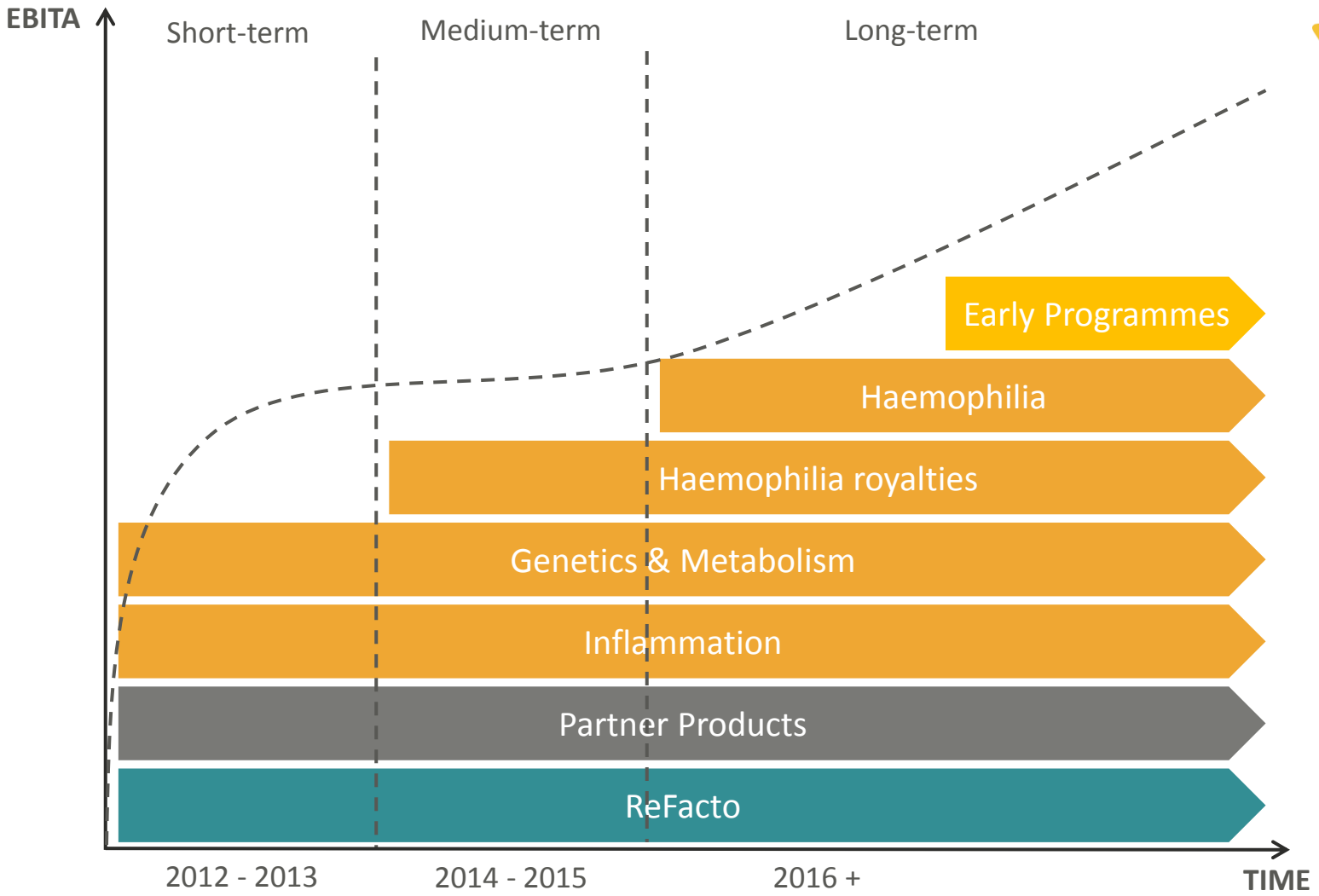
# Building Our Future

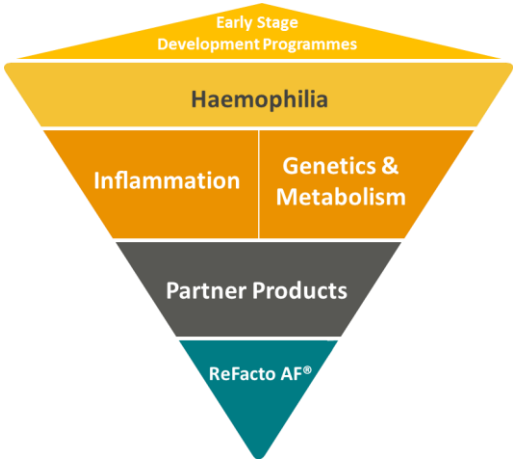
1. Diverse, growing, and profitable base business focused on rare diseases

2. First to market, long-lasting haemophilia factors will potentially enter exclusive Sobi territories in the near term
(current market size is USD 3.7 Billion)

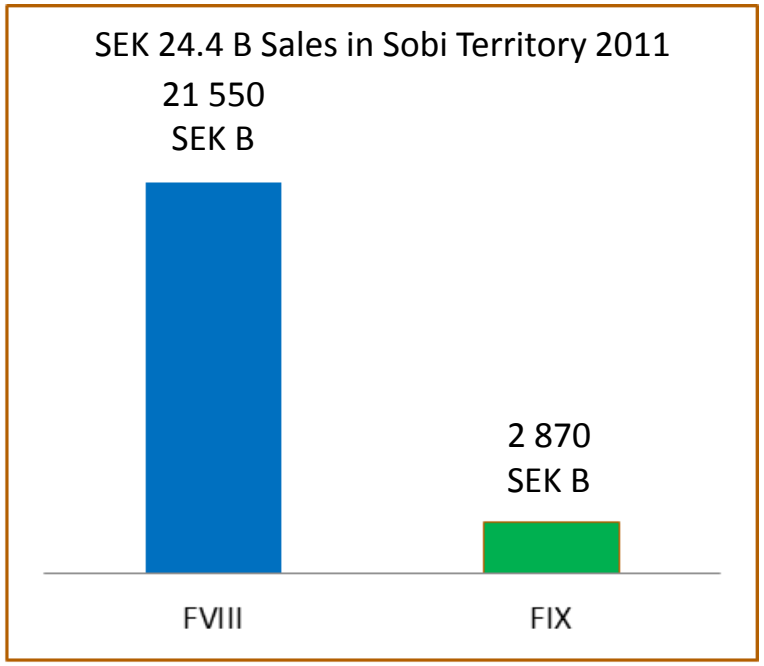
3. Pipeline of rare disease biologics

# Near-Term First to Market Haemophilia Franchise



# Haemophilia A+B Sales in Sobi Territory

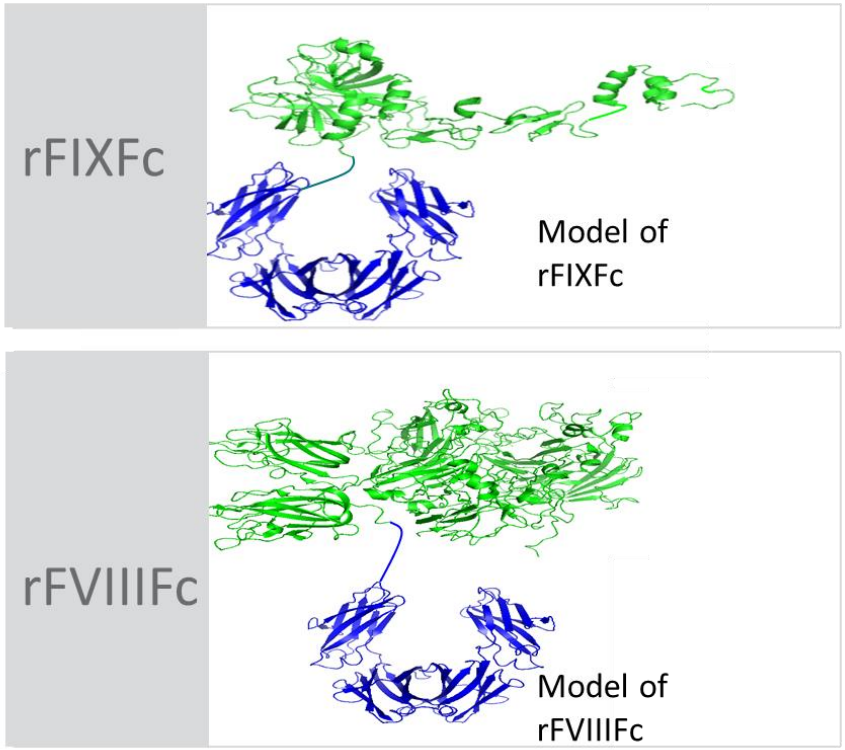



Source: Marketing Research Bureau 2011
(http://www.marketingresearchbureau.com)


- Albania
- Algeria
- Andorra
- Armenia
- Austria
- Azerbaijan
- Bahrain
- Belarus
- Belgium
- Bosnia Herzegovina
- Bulgaria
- Croatia
- Cyprus
- Czech Republic
- Denmark
- Djibouti
- Egypt
- Estonia
- Finland
- Former Yugoslav Republic of Macedonia
- France
- Georgia
- Germany
- Greece
- Hungary
- Iceland
- Iran
- Iraq
- Ireland
- Italy
- Jordan
- Kuwait
- Latvia
- Lebanon
- Libya
- Liechtenstein
- Lithuania
- Luxembourg
- Malta
- Mauritania
- Moldova
- Monaco
- Morocco
- Norway
- Oman
- Poland
- Portugal
- Qatar
- Romania
- Russia
- San Marino
- Saudi Arabia
- Serbia & Montenegro
- Slovakia
- Slovenia
- Somalia
- Spain
- Sudan
- Sweden
- Switzerland
- Syria
- The Netherlands
- Tunisia
- Turkey
- UAE
- Ukraine
- United Kingdom
- Vatican City
- Yemen

*US$ 1 = SEK 6.53 (Exchange rate 2 May, 2014)*

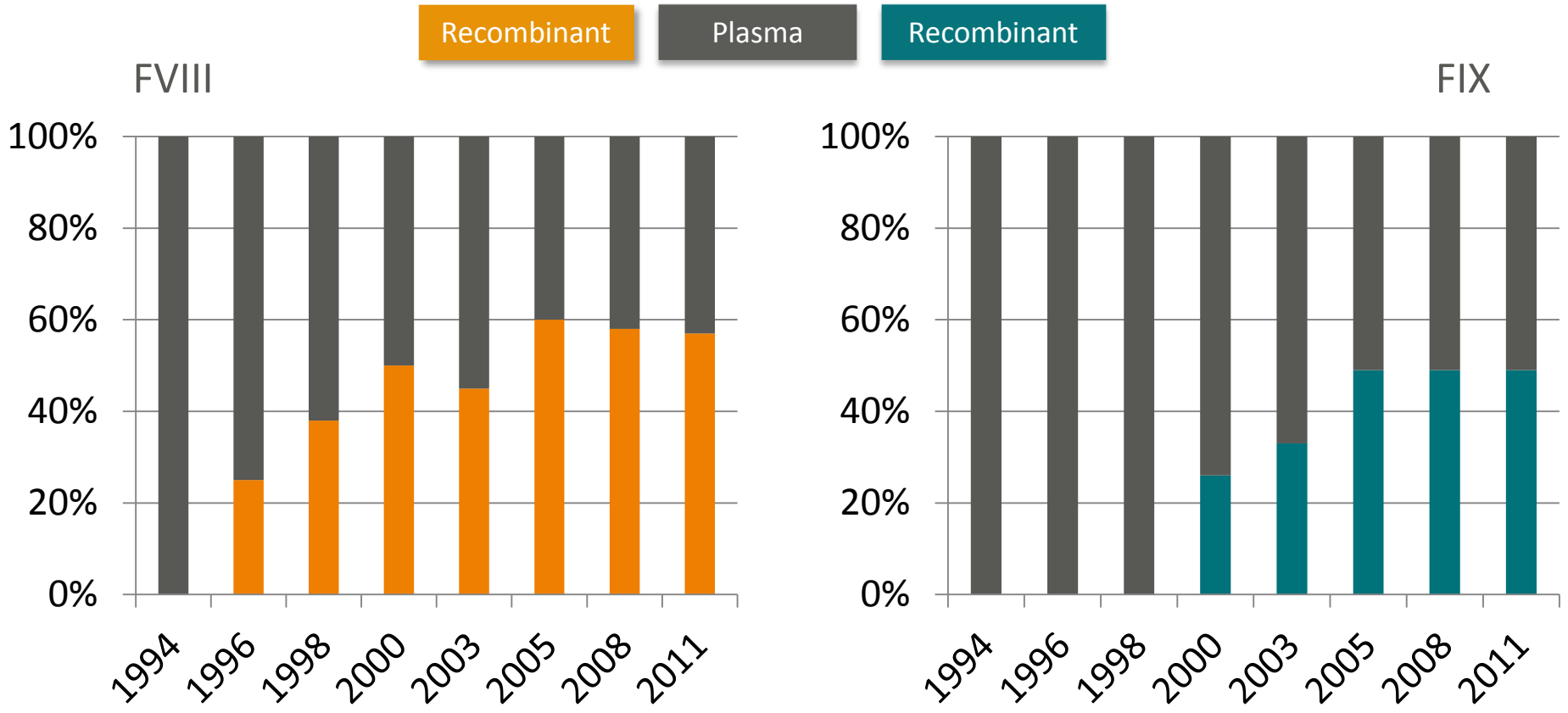
# Fc Fusion Technology: Long-acting Clotting Factors




- Clotting factors must be infused 2-4 times per week intravenously
- Burden of therapy is a major factor influencing patient compliance and outcomes
- Our long-acting factors can reduce annual injections by 50 – 75 per year
- Longer-lasting clotting factors are the most desired innovation by people with Haemophilia today

# History: Introduction of Recombinant Clotting Factors

Market share % by volume, the EU Market:



# Fewer than 100 Haemophilia Reference Centres

# Expected Haemophilia Timelines

## Factor 8

**US**
Biogen Idec/Sobi
FDA approval expected MY 2014

**EU**
Biogen Idec/Sobi
EMA approval potentially YE 2015

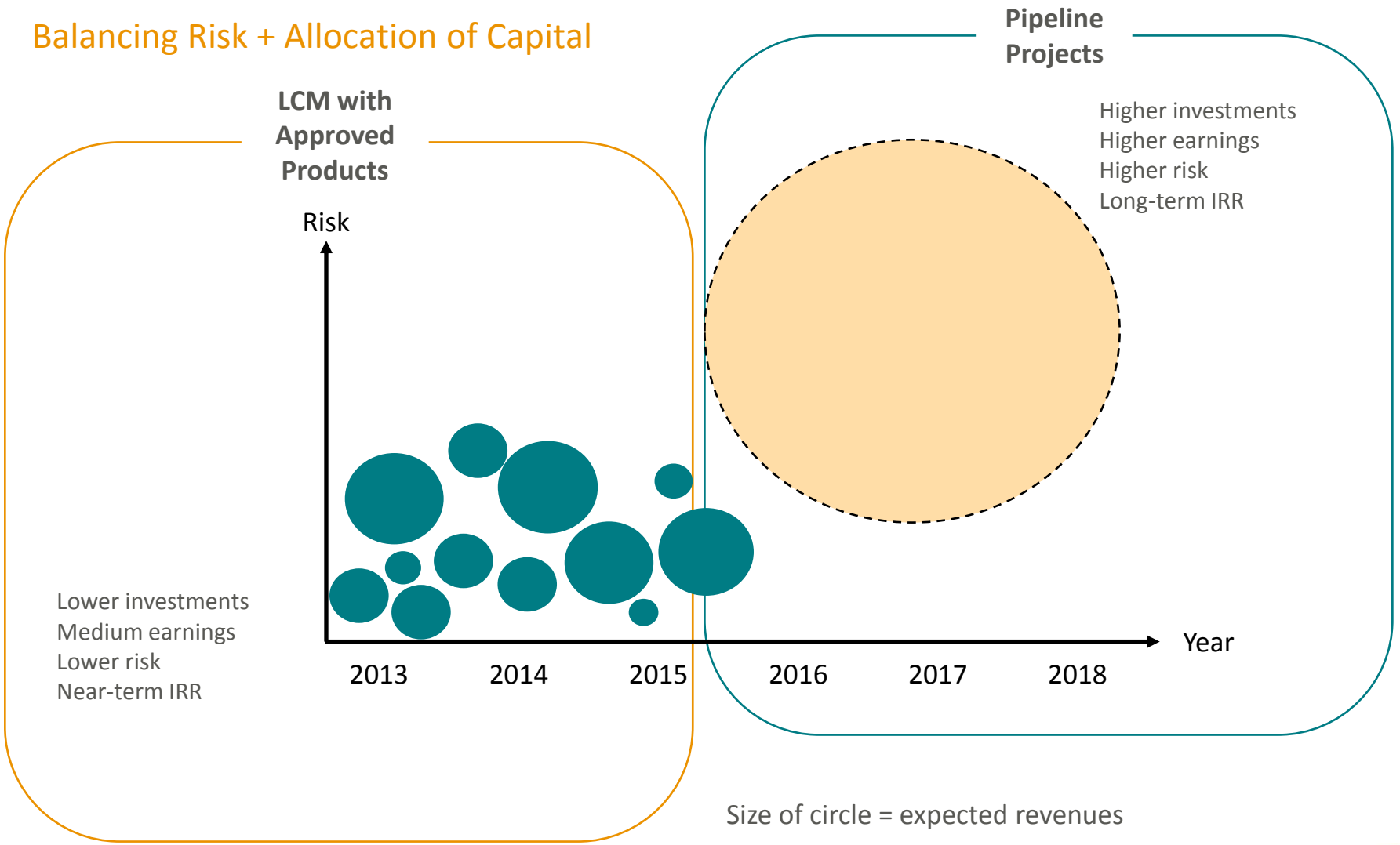
## Factor 9

**US**
Biogen Idec/Sobi
FDA approval 28 March 2014

**EU**
Biogen Idec/Sobi
EMA approval potentially H2 2016

# Pipeline of Rare Disease Biologics

## Balancing Risk + Allocation of Capital



# Pipeline of Rare Disease Biologics

| Indication | Project | Partner | Pre-Clin. | Phase 1 | Phase 2 | Phase 3 | Launch |
|---|---|---|---|---|---|---|---|
| Hemophilia A | rFVIIIFc | biogen idec. | | | | | |
| Hemophilia B | rFIXFc | biogen idec. | | | | | |
| Oral Mucositis in Head & Neck Cancer | Kepivance | sobi | | | | | |
| Hereditary Tyrosinaemia type 1 | Orfadin Liquid | sobi | | | | | |
| Hereditary Tyrosinaemia type 1 | Orfadin 20mg capsule | sobi | | | | | |
| Alkaptonuria | Orfadin | AKU alkaptonuria society | | | | | |
| Complement Factor C5 | SOBI002 | AFFIBODY | | | | | |
| Enzyme Replacement Therapy | SOBI003 | sobi | | | | | |
| IL-1-driven disease | IL-1 Affibody | AFFIBODY | | | | | |

Pipeline Projects | LCM with Approved Products | Product about to be launched



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# R&D Pipeline 2014

| Indication | Project | Partner | Pre-Clin. | Phase 1 | Phase 2 | Phase 3 | Launch |
|---|---|---|---|---|---|---|---|
| Hemophilia A | rFVIIIFc | biogen idec. | | | | | |
| Hemophilia B | rFIXFc | biogen idec. | | | | | |
| Oral Mucositis in Head & Neck Cancer | Kepivance | sobi | | | | | |
| Hereditary Tyrosinaemia type 1 | Orfadin Liquid | sobi | | | | | |
| Hereditary Tyrosinaemia type 1 | Orfadin 20mg capsule | sobi | | | | | |
| Alkaptonuria | Orfadin | AKU alkaptonuria society | | | | | |
| Complement Factor C5 | SOBI002 | AFFIBODY | | | | | |
| Enzyme Replacement Therapy | SOBI003 | sobi | | | | | |
| IL-1-driven disease | IL-1 Affibody | AFFIBODY | | | | | |

Pipeline Projects | LCM with Approved Products | Product about to be launched



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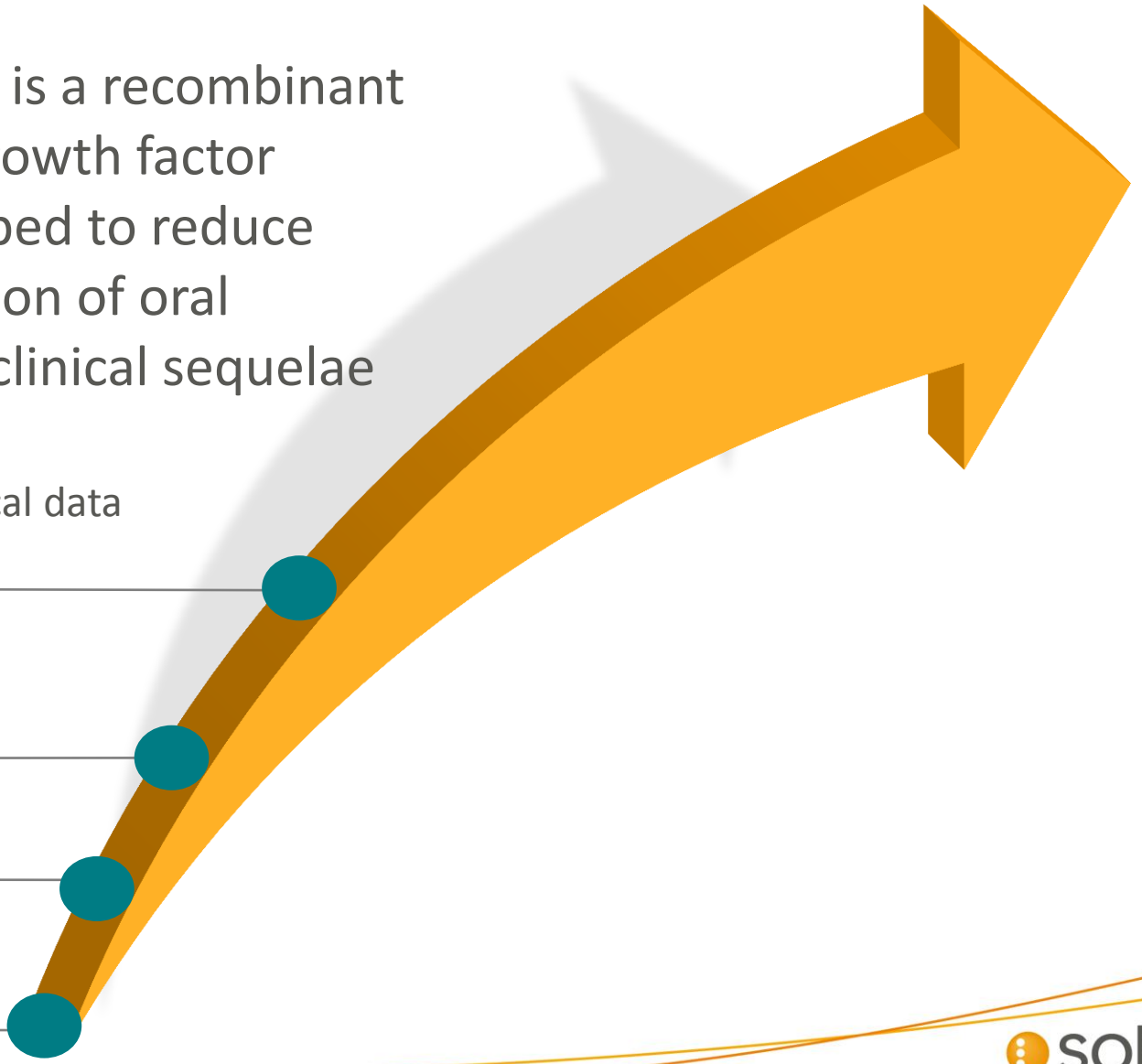
# Kepivance Background

- Kepivance (palifermin) is a recombinant human keratinocyte growth factor
- Palifermin was developed to reduce the severity and duration of oral mucositis and related clinical sequelae



2013 rights to additional clinical data for Kepivance from Amgen

2008 proprietary product, acquired from Amgen

2005 emea

2004 FDA



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# Kepivance: Current + Potential Future Indication

**Current Indication:**

Bone Marrow Transplant

to decrease the incidence and duration of severe oral mucositis in patients with hematologic malignancies receiving myelotoxic therapy requiring hematopoietic stem cell support

There are approximately 10,000 – 15,000 addressable patients in the US each year

**Proposed Indication:**

Head and Neck Cancers

to decrease the incidence and duration of severe oral mucositis in patients with unresected locally advanced patients and for high risk resected patients receiving chemoradiation therapy

52,500 new cases of Head and Neck cancer in US 2013; ~ 35,000 patients could be addressable

# Generation of Affibody C5 Inhibitors

**2011: Lead optimization**

PK/PD, process feasibility, preclinical immunogenicity assessment

**2010: Affinity maturation**

Library design, phage display selection and screening

**2010: Functional characterization**

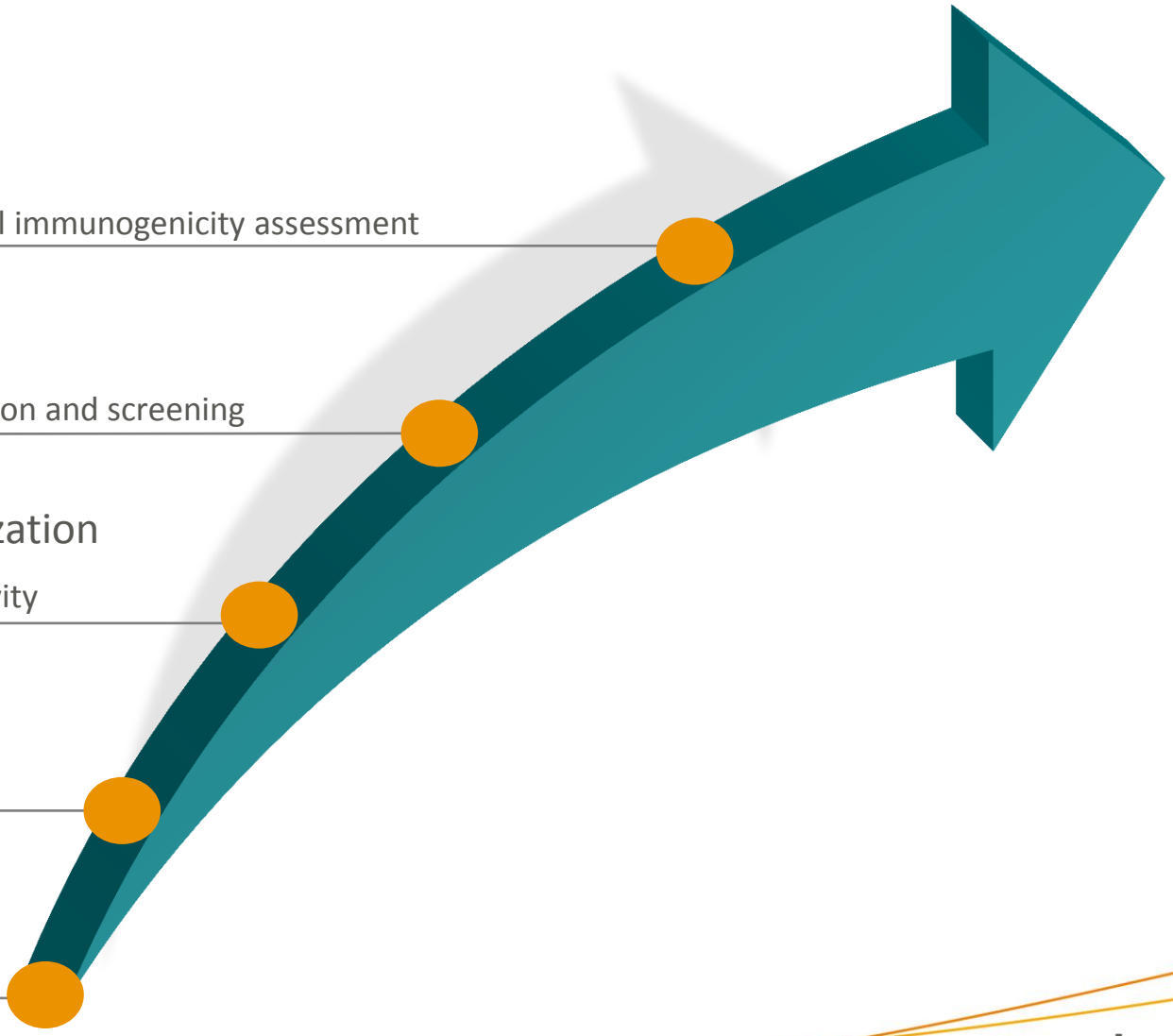
*In vitro*, *in vivo*, species cross reactivity

**2009: Affibody screening**

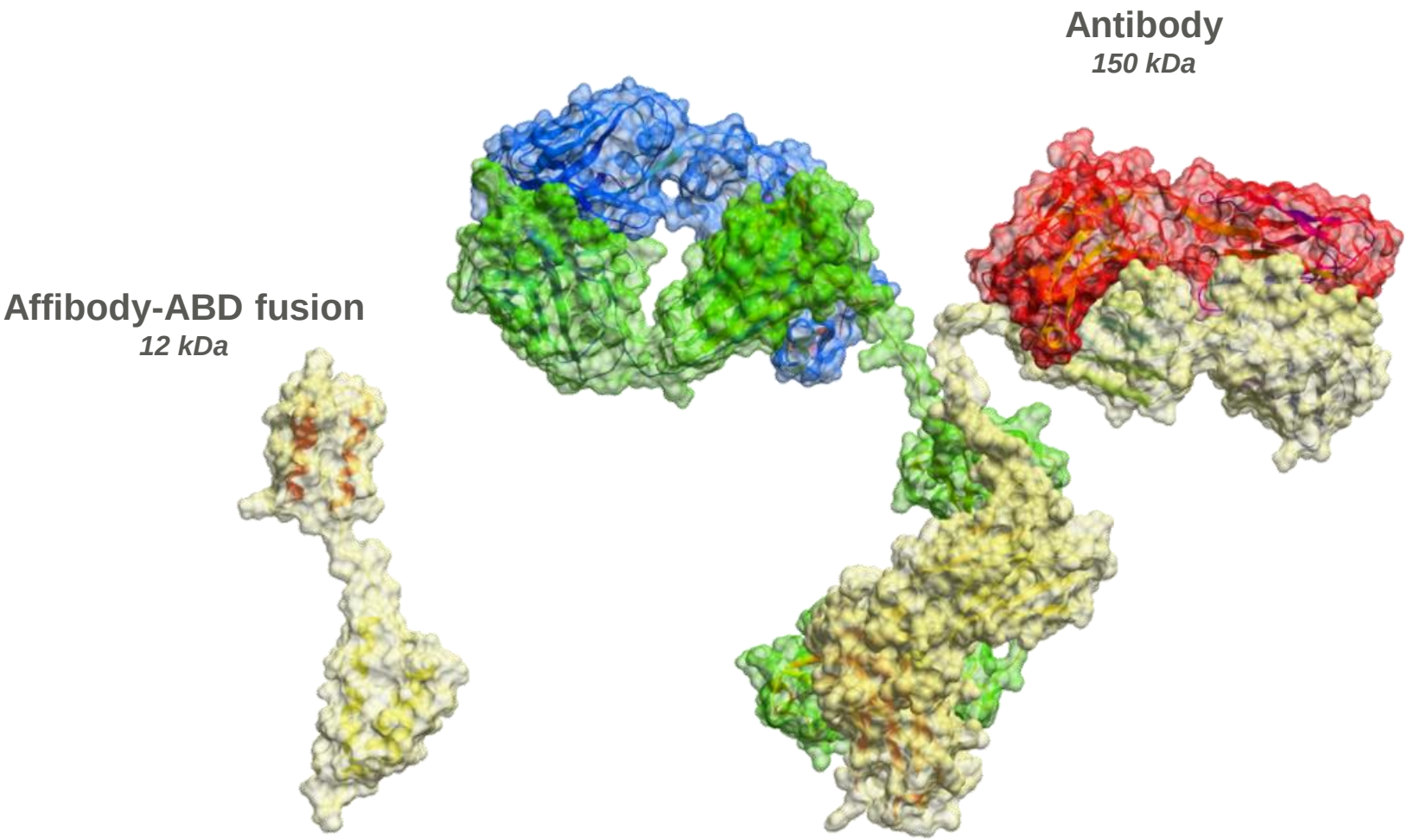
ELISA, functional assays

**2009: Affibody selection**

Phage display



# The Size of an Affibody-ABD Fusion Protein Is Less Than 10 per cent of an Antibody



# Strategic Priorities

1. **Near-term** focus on growth in key therapeutic areas, with sustainable positive cash flow from operations.

We are here

2. **Medium-term** investments to ensure successful commercialisation of our late-stage pipeline.

3. **Long-term** growth will come organically and through acquisitions in key therapeutic areas.

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# Investing in our abilities

**PRESS RELEASE**
Stockholm, 12 November, 2013

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Pioneer in Rare Diseases

**Sobi appoints Philip Wood Vice President and Commercial Therapeutic Area Head Haemophilia**

Swedish Orphan Biovitrum AB (publ) (Sobi) today announced that Philip Wood has been appointed Vice President and Commercial Therapeutic Area Head Haemophilia.

Philip Wood joined Sobi in March 2012 as the Global Strategic Lead for the company's Haemophilia A team,

Dr. Mitchev has been appointed Vice President and Medical Therapeutic Area Head, Haemophilia. He earned

After a thorough search process supported by a senior Executive Search firm, Mats-Olof Wallin was selected



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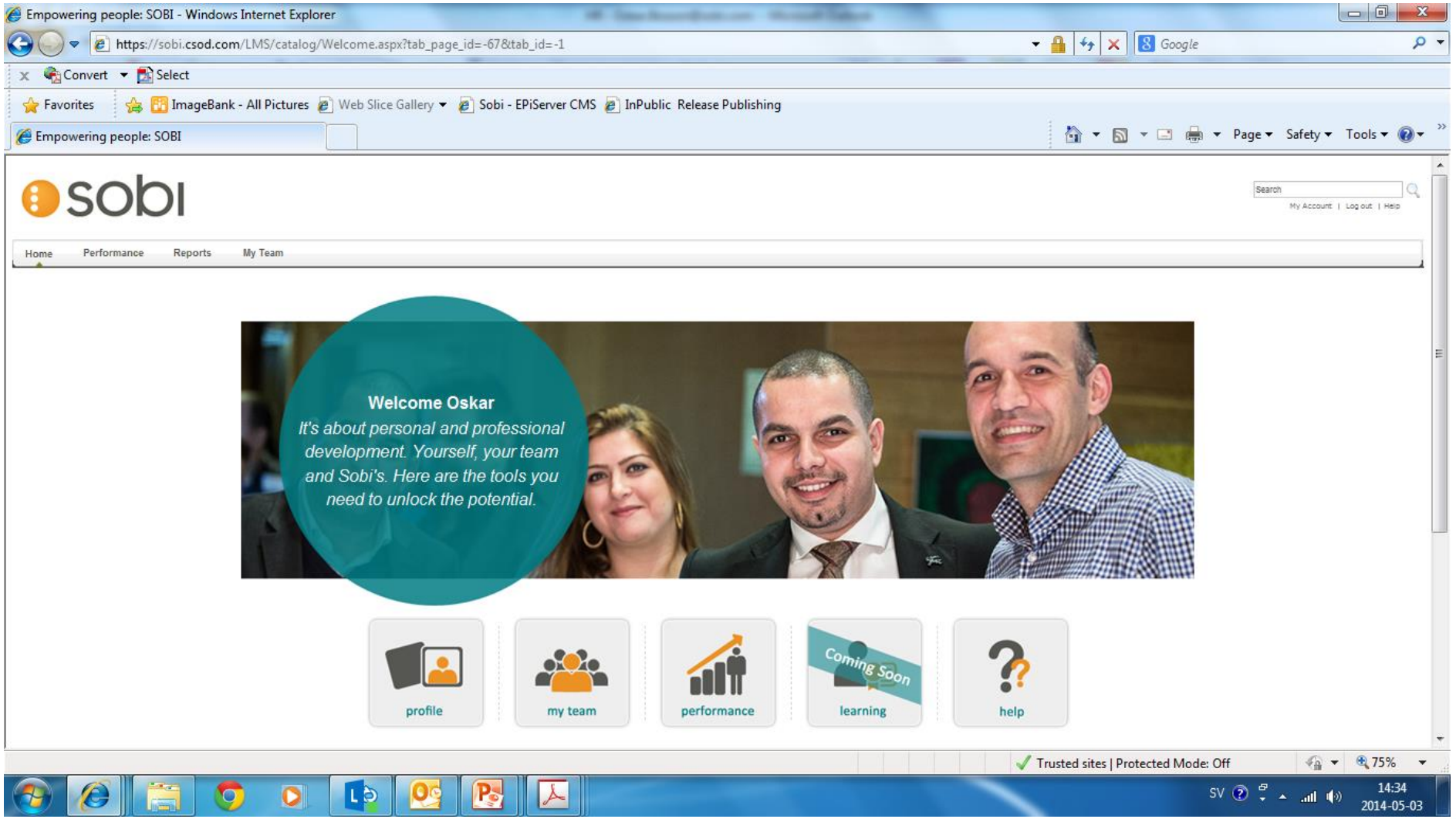
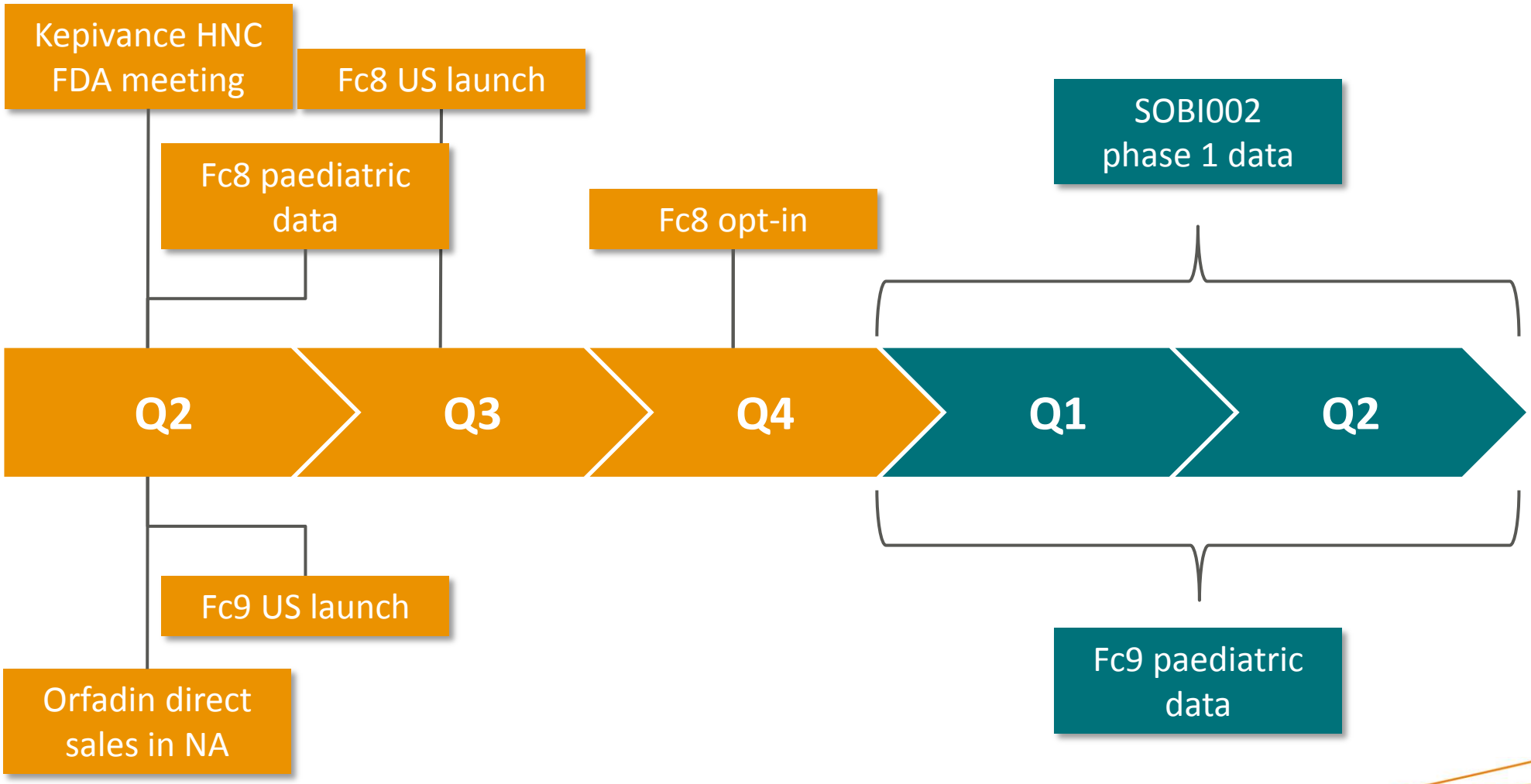
# Investing in our people



# Key Events Calendar 2014 - 2015*




* *Timing for all events is indicative*

# Summary

1. Diversified commercial portfolio focused on **improving cash flow and profitability**

2. Working to efficiently commercialize **our proprietary innovative medicines** for rare disease patients globally

3. Business model oriented to **building value through partnerships** from global early stage biologics development to late stage specialty distribution in Europe

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