

## PRESS RELEASE

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### **Sobi™ to present new interim ASPIRE data at EAHAD congress suggesting that patients can reduce bleeding rates with long-term Elocta® treatment**

New interim results from ASPIRE, the ongoing long-term extension study of Elocta® (efmoroctocog alfa), marketed by Biogen as Eloctate® (Antihemophilic Factor (Recombinant), Fc Fusion Protein) in the United States, suggest that patients can maintain prolonged protection against bleeding episodes at extended prophylactic dosing intervals. The data will be presented by [Swedish Orphan Biovitrum AB \(publ\)](#) (Sobi) (STO: SOBI) and [Biogen](#) (NASDAQ: BIIB) at the 9<sup>th</sup> Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD), taking place in Malmö, Sweden, 3-5 February, 2016.

In total 13 posters will be presented by the two companies, including results from a European retrospective study of the current treatment practices of haemophilia A and B which emphasises the need for additional innovation in haemophilia. Further data will be presented that show how Elocta may improve joint health in people with haemophilia A.

“The interim data from our long-term Elocta extension study, ASPIRE, show that the overall annualised bleeding rates were maintained or even further reduced with long-term prophylactic treatment compared to the rates seen in the pivotal studies”, said Dr Krassimir Mitchev, MD, PhD, Vice President, Medical Therapeutic Area Head Haemophilia at Sobi. “In addition, the results of our European observational retrospective study show that people with haemophilia on conventional prophylactic treatment still experience breakthrough bleeds. The results suggest that there is an opportunity for further improvement of the treatment of haemophilia.”

ASPIRE is an ongoing open-label, non-randomised, multi-year extension study for people who completed the pivotal, phase 3 A-LONG or Kids A-LONG studies. A-LONG and Kids A-LONG demonstrated the efficacy, safety and pharmacokinetics of Elocta in previously treated males 12 years of age and older, and children less than 12 years old, respectively, with severe haemophilia A.

Dr Mitchev continued: “These interim results from ASPIRE highlight the value of extended half-life therapy in managing bleeds and further support the safety and efficacy profile of Elocta, the first meaningful treatment advance for people with haemophilia A in the EU in nearly 20 years. The data adds to the body of robust clinical data for Elocta over the longest clinical observation period of any extended half-life therapy to date.”

The titles of the poster presentations are as follows:

*Haemophilia overall:*

- Adherence to treatment in haemophilia: a comparison of conventional and prolonged half-life therapies; *P115*
- A European Retrospective Study of the Current Treatment Practice of Haemophilia A; *P059*
- A European Retrospective Study of the Current Treatment Practice of Haemophilia B; *P062*

*Elocta®/Eloctate® (rFVIII Fc):*

- Long-Term Safety and Efficacy of Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) in Adults and Adolescents with Severe Haemophilia A: An Updated Interim Analysis of the ASPIRE Study; *P070*
- Second Interim Analysis of the ASPIRE Study Evaluating Long-Term Safety and Efficacy of Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) in Children with Severe Haemophilia A; *P072*
- Modified Haemophilia Joint Health Scores (mHJHS) Outcomes With Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) Prophylaxis in Subjects With Severe Haemophilia A; *P075*
- Long-Term Efficacy of Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) Prophylaxis in Paediatric, Adolescent, and Adult Subjects with Target Joints and Severe Haemophilia A; *P003*
- Low Bleeding Rates With Increase or Maintenance of Physical Activity in Patients Treated With Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) in the A-LONG and Kids A-LONG Studies; *P002*
- The cost-effectiveness of rFVIII Fc in a Swedish setting; *P086*

*Alprolix® (rFIX Fc):*

- Effect of Sampling Duration on Pharmacokinetic (PK) Parameters of Recombinant Factor IX Fc Fusion Protein (rFIX Fc) in the Phase 3 B-LONG Study; *P040*
- Extended-Interval Prophylaxis With Recombinant Factor IX Fc Fusion Protein (rFIX Fc) in Adults/Adolescents With Haemophilia B: Interim Results of the B-YOND Extension Study; *P044*
- Clinical Outcomes in Children With Haemophilia B Treated Long Term With Recombinant Factor IX Fc Fusion Protein (rFIX Fc) Prophylaxis: Interim Results of the B-YOND Extension Study; *P045*
- Low Bleeding Rates With Increase or Maintenance of Physical Activity in Patients Treated With Recombinant Factor IX Fc Fusion Protein (rFIX Fc) in the B-LONG and Kids B-LONG Studies; *P001*

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**About Elocta®**

Elocta (efmoroctocog alfa) is the first recombinant clotting factor VIII therapy in the EU that offers an extended half-life in the body. It is indicated for the treatment and prophylaxis of bleeding episodes in patients with haemophilia A (factor VIII deficiency) and can be used by people of all ages. It is also known as Eloctate® (Antihemophilic Factor (Recombinant), Fc Fusion Protein) in Australia, Canada, Japan, New Zealand and the U.S., where it is approved for the treatment of haemophilia A.

Elocta was developed by fusing B-domain deleted factor VIII to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body). This enables Elocta to utilise a naturally occurring pathway to prolong the time the therapy remains in the body. While Fc fusion technology has been used in other therapies for more than 15 years, Sobi and Biogen are the first companies to utilise it in the treatment of haemophilia. As with any infused protein, allergic type hypersensitivity reactions and development of inhibitors may occur following administration of Elocta. For full prescribing information visit [www.elocta.com](http://www.elocta.com).

**About haemophilia A and B**

Haemophilia is a rare, genetic disorder in which the ability of a person's blood to clot is impaired. Haemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. Haemophilia B occurs in about one in 25,000 male births annually, and more rarely in females. Worldwide, it is estimated that more than 400,000 people are living with haemophilia. Haemophilia A is caused by having substantially reduced or no factor VIII activity, while haemophilia B is caused by having substantially reduced or no factor IX activity; factor VIII and factor IX are needed for normal blood clotting.

People with haemophilia A or B experience prolonged bleeding episodes that can cause pain, irreversible joint damage and life-threatening haemorrhages. Prophylactic infusions of factor VIII or IX can temporarily replace the missing clotting factors that are needed to control bleeding and prevent new bleeding episodes.<sup>i</sup> The World Federation of Hemophilia recommends that prophylaxis be the goal of therapy because it may prevent bleeding and joint destruction. As a result, regular prophylactic treatment may slow progression of joint disease and may improve quality of life.<sup>ii</sup>

**About the Biogen/Sobi collaboration**

Sobi and Biogen are collaboration partners in the development and commercialisation of Elocta for haemophilia A, which is also known as Eloctate® (Antihemophilic Factor (Recombinant), Fc Fusion Protein) in Australia, Canada, Japan, New Zealand and the U.S., where it is approved for the treatment of haemophilia A. Sobi holds final development and commercialisation rights in pre-specified territories, which include Europe, North Africa, Russia and certain countries in the Middle East. Biogen leads development and manufacturing of the product and holds commercialisation rights in North America and all other regions in the world outside of the Sobi territory.

**About Sobi**

Sobi is an international specialty healthcare company dedicated to rare diseases. Sobi's mission is to develop and deliver innovative therapies and services to improve the lives of patients. The product portfolio is primarily focused on Haemophilia, Inflammation and Genetic diseases. Sobi also markets a portfolio of specialty and rare disease products for partner companies across Europe, the Middle East, North Africa and Russia. Sobi is a pioneer in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2014, Sobi had total revenues of SEK 2.6 billion (USD 380 M) and about 600 employees. The share (STO: SOBI) is listed on NASDAQ OMX Stockholm. More information is available at [www.sobi.com](http://www.sobi.com).

**For more information please contact**

## Media relations

Oskar Bosson, Head of Communications

+46 70 410 71 80

[oskar.bosson@sobi.com](mailto:oskar.bosson@sobi.com)

## Investor relations

Jörgen Winroth, Vice President, Head of Investor Relations

+1 347-224-0819, +1 212-579-0506, +46 8 697 2135

[jorgen.winroth@sobi.com](mailto:jorgen.winroth@sobi.com)

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<sup>i</sup> Hemophilia Federation of America. What is Hemophilia? Available at: <http://www.hemophiliafed.org/bleeding-disorders/hemophilia/treatment/>.  
Accessed: November 15

<sup>ii</sup> Guideline for the management of hemophilia, World Federation of Hemophilia, 2nd edition, <http://www1.wfh.org/publication/files/pdf-1472.pdf>.  
Accessed: December 2015