

## **PRESS RELEASE**

Stockholm, Sweden, and Cambridge, Mass., USA, 3 December 2016

### **Data reinforcing the long-term safety and efficacy of extended half-life haemophilia therapies Elocta® and Alprolix® highlighted at 58th ASH meeting**

*New longitudinal data shows long-term prophylactic use of Elocta/Eloctate resulted in effective target joint resolution and improved quality of life measures*

[Biogen](#) (NASDAQ: BIIB) and [Swedish Orphan Biovitrum AB \(publ\)](#) (Sobi™) (STO: SOBI) will present new data, including updated longitudinal safety and efficacy findings from phase 3 and extension studies, on the companies' extended half-life therapies, Elocta® (efmoroctocog alfa) (marketed as Eloctate® in the United States and certain other countries) for haemophilia A and Alprolix® (eftrenonacog alfa) for haemophilia B, at the 58th American Society of Hematology (ASH) Annual Meeting & Exposition in San Diego, California, from 3-6 December.

The presentations include efficacy data, which show low target joint annual bleeding rates and effective target joint resolution ( $\leq 2$  spontaneous bleeding episodes over one year) in pediatric, adolescent and adult patients on long-term prophylaxis with Elocta/Eloctate. Target joints occur when people with haemophilia experience frequent bleeds in the same joint, and can lead to chronic joint disease. An 18% improvement in haemophilia-related quality of life measures (Haem-A-QOL) was seen in adolescents and adults who experienced target joint resolution with prophylactic treatment with Elocta/Eloctate as compared to baseline measurements at phase 3 study entry, with the most impact ( $\geq 20\%$ ) in areas such as physical health, sports and leisure, and work and school.

Biogen will also present preclinical data on recombinant FIXFc-XTEN, a fusion protein being investigated for once-weekly, subcutaneous treatment of haemophilia B. The rFIXFc-XTEN program is currently being developed solely by Biogen and utilizes XTEN® technology licensed from Amunix. Depending on Biogen's development activities and other factors, it is subject to an opt-in right by Sobi in the future.

"The data presented at ASH, coupled with the real-world experience of Eloctate/Elocta and Alprolix, may help patients, clinicians and policymakers better understand the long-term safety and sustained efficacy profile for these therapies," said Maha Radhakrishnan, MD, vice president, medical, at Biogen and head of medical at Bioverativ Inc., a spin-off of Biogen's haemophilia business that is on track to launch in early 2017. "We also remain deeply committed to developing new treatments that can make a meaningful impact in the lives of people with haemophilia."

“We are encouraged to see data that show Elocta/Eloctate can improve the quality of life for people with haemophilia A,” said Krassimir Mitchev, MD, PhD, vice president and medical therapeutic area head of Haemophilia at Sobi. “Sobi, together with Biogen, is strongly committed to ensuring sustainable access to Eloctate/Elocta for people living with haemophilia A across our respective markets. “

Elocta/Eloctate and Alprolix have more than two years of real-world experience and are the only haemophilia therapies developed using Fc fusion technology, which enables them to use the body's natural pathway to prolong the time the therapies remain in the body.

These therapies and rFIXFc-XTEN are part of Biogen's haemophilia business, which Biogen plans to spin off into Bioverativ, an independent, public company focused on the discovery, research, development and commercialization of treatments for haemophilia and other rare blood disorders. Bioverativ will continue to collaborate with Sobi on their joint development programmes.

Selected presentation information:

*Elocta/Eloctate and Alprolix focused*

- Longitudinal Analysis of Long-term Safety and Efficacy of Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) in Adults/Adolescents with Severe Hemophilia A – Session: 322, Disorders of Coagulation or Fibrinolysis: Poster I - #1413 - Saturday, December 3, 5:30-7:30 PM PST
- Longitudinal Analysis of Long-term Safety and Efficacy of Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) in Previously Treated Children with Severe Hemophilia A – Session: 322, Disorders of Coagulation or Fibrinolysis: Poster I - #1414 - Saturday, December 3, 5:30-7:30 PM PST
- Clinical Outcomes in Adults/Adolescents with Hemophilia B Treated Long Term with Recombinant Factor IX Fc Fusion Protein (rFIX Fc) Prophylaxis: Interim Results of the B-YOND Extension Study – Session: 322, Disorders of Coagulation or Fibrinolysis: Poster I - #1416 - Saturday, December 3, 5:30-7:30 PM PST
- Long-term Efficacy and Quality of Life With Recombinant Factor VIII Fc Fusion Protein (rFVIII Fc) Prophylaxis in Pediatric, Adolescent, and Adult Subjects with Target Joints and Severe Hemophilia A – Session: 322, Disorders of Coagulation or Fibrinolysis: Poster III - #3791 – Monday, December 5, 6:00-8:00 PM PST

*Biogen's rFIXFc-XTEN:*

Evaluation of rFIXFc-XTEN bleeding efficacy in Hemophilia-B mouse models – Session: 321, Blood Coagulation and Fibrinolytic Factors: Poster III - #3757 – Monday, December 5, 6:00-8:00 PM PST

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### **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. The World Federation of Hemophilia estimates that approximately 180 000 people are currently diagnosed with haemophilia A and B world-wide<sup>[viii]</sup>.

People with haemophilia A or B experience bleeding episodes that can cause pain, irreversible joint damage and life-threatening haemorrhages. Prophylactic injections of factor VIII or IX can temporarily replace the clotting factors that are needed to control bleeding and prevent new bleeding episodes<sup>[viii]</sup>. The World Federation of Hemophilia recommends prophylaxis as the optimal therapy as it can prevent bleedings and joint destruction<sup>[ix]</sup>.

### **About Elocta®/Eloctate®**

Elocta® (efmorotocog alfa), is a recombinant clotting factor VIII therapy developed for haemophilia A with prolonged circulation in the body using Fc fusion technology. It is engineered by fusing factor VIII to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body), enabling Elocta to use a naturally occurring pathway to extend the time the therapy remains in the body. While Fc fusion technology has been used for more than 15 years, Sobi and Biogen have optimized the technology and are the first companies to utilize it in the treatment of haemophilia. Elocta is manufactured in a human cell line, using an environment free of animal and human additives.

Elocta is approved for the treatment of haemophilia A in the European Union, Switzerland, Iceland, Liechtenstein, Norway and Kuwait, marketed by Sobi and in the United States, Japan, Canada, Australia, New Zealand, Brazil, and other countries as Eloctate and Biogen has the marketing rights in these regions.

As with any factor replacement therapy, allergic-type hypersensitivity reactions and development of inhibitors may occur in the treatment of hemophilia A. Inhibitor development has been observed with Eloctate/Elocta, including in previously untreated patients. For more information, please see the full [U.S. prescribing information](#) for Eloctate. Note that the indication for previously untreated patients is not included in the [EU Product Information](#) for Elocta.

### **About Alprolix®**

Alprolix® (eftrenonacog alfa), is a recombinant clotting factor therapy developed for haemophilia B using Fc fusion technology to prolong circulation in the body. It is engineered by fusing factor IX to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body), enabling Alprolix to use a naturally occurring pathway to extend the time the therapy remains in the body (half-life). While Fc fusion technology has been used for more than 15 years, Biogen and Sobi have optimized the technology and are the first companies to utilize it in the treatment of haemophilia. Alprolix is manufactured using a human cell line in an environment free of animal and human additives.

Alprolix is approved for the treatment of haemophilia B the European Union, Iceland, Liechtenstein, Schweiz and Norway marketed by Sobi, as well as in the United States, Canada, Japan, Australia, New Zealand, Brazil and other countries and Biogen has the marketing rights in these regions.

Allergic-type hypersensitivity reactions and development of inhibitors have been observed with Alprolix in the treatment of hemophilia B, including in previously untreated patients. For more information, please see the full [U.S. prescribing information](#) for Alprolix. Note that the indication for previously untreated patients is not included in the [EU Product Information](#).

### **About Biogen**

Through cutting-edge science and medicine, Biogen discovers, develops and delivers worldwide innovative therapies for people living with serious neurological, autoimmune and rare diseases. Founded in 1978, Biogen is one of the world's oldest independent biotechnology companies, and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies. For more information, please visit [www.biogen.com](http://www.biogen.com). Follow us on [Twitter](#).

### **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 across Europe, the Middle East, North Africa and Russia for partner companies. Sobi is a pioneer in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2015, Sobi had total revenues of SEK 3.2 billion (USD 385 M) and about 700 employees. The share (STO: SOBI) is listed on Nasdaq Stockholm. More information is available at [www.sobi.com](http://www.sobi.com).

### **About the Biogen and Sobi Collaboration**

Biogen and Sobi collaborate on the development and commercialisation of Elocta/Eloctate and Alprolix. Sobi has final development and commercialisation rights in the Sobi territory (essentially Europe, North Africa, Russia and most Middle Eastern markets). Biogen has manufacturing responsibility for Eloctate/Elocta and Alprolix and has final development and commercialisation rights in North America and all other regions in the world excluding the Sobi territory.

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<sup>[vii]</sup> World Federation of Hemophilia, Annual Global Survey 2015, published in October 2016. Available at: <http://www.wfh.org/en/data-collection>

<sup>[viii]</sup> World Federation of Hemophilia. About Bleeding Disorders – Frequently Asked Questions. Available at: [http://www.wfh.org/en/page.aspx?pid=637#Difference\\_A\\_B](http://www.wfh.org/en/page.aspx?pid=637#Difference_A_B). Accessed on: June 17, 2016.

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