PRESS RELEASE

Stockholm, Sweden, 7 February 2018

Time to elevate protection in haemophilia care: New data from Sobi™

Swedish Orphan Biovitrum AB (publ) (Sobi™) will present new evidence at the upcoming 11th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD), in Madrid, Spain, 7-9 February 2018. These data mark the company´s long-term commitment to elevate haemophilia management and expand the available clinical evidence for Elocta® and Alprolix®, demonstrating the need to rethink the current concept of protection, and advance the standard of haemophilia care for people with haemophilia A and B.

“Bleeding control and prevention remain the cornerstone of haemophilia care, however new findings advocate for a more holistic view on protection that can never compromise on safety. This is built on prevention and treatment of bleeds, joint health, freedom of pain, as well as patient´s preferences, physical activity and overall quality of life”, said Philip Wood, Head of Haemophilia at Sobi. “This may encourage the haemophilia community to re-think the current definition of protection and evolve the standard of care”.

Sobi is dedicating significant efforts to generating both clinical and real-world evidence and translate this holistic perspective on protection into reality for the haemophilia community; from safety and clinical outcomes, to quality of life and sustainable access, to more effective treatments for people with haemophilia A and B.

In line with this, one of the posters presented by Sobi includes the first real world comparison of prophylactic treatment in patients with haemophilia A, before and after switching from conventional rFVIII therapy, to Elocta (rFVIIIFc). The data, gathered from the UK National Haemophilia Database, demonstrates a significant reduction of injection frequency and clotting factor consumption and provides further evidence that extended half-life rFVIII products can reduce treatment burden.

Sobi will also present the results of pharmacokinetic simulations for different prophylactic treatments and dosing regimens for haemophilia A in Germany, and the economic impact thereof. The simulation suggests that by using rFVIIIFc instead of conventional rFVIII therapy it is possible to achieve better bleed protection and extend the dosing interval, lower factor consumption and reduce healthcare costs.

“Protection for people with haemophilia should always be made a priority without compromising on safety”, said MD, PhD, Armin Reininger, Head of Medical and Scientific Affairs, at Sobi.

The development of inhibitors is one of the greatest challenges in haemophilia treatment and eradication of the inhibitor is considered the current standard of care. Sobi and its collaborator Bioverativ are committed to further investigate Elocta in the Immune Tolerance Induction (ITI) setting. The poor quality of life (QoL) of severe haemophilia patients with inhibitors and QoL improvement following eradication of inhibitors via ITI

are well documented in the literature. The design of ReITIrate, the first prospective study of ITI with an extended half-life FVIII product in patients not responding to previous ITI attempts, and the study design for verITI-8, a global interventional study evaluating rFVIIIFc for initial ITI therapy, could further contribute to the growing evidence of the potential use of Elocta in this setting.

“We are committed to providing the haemophilia community with an increasing amount of scientific evidence that will help in advancing haemophilia care. This includes taking a more holistic approach, understanding how we can provide protection beyond bleeds”, said MD, PhD, Armin Reininger, Head of Medical and Scientific Affairs, at Sobi. “We envision a world where people with haemophilia can live a normal life, without any constraints or compromises”.

The posters which have been accepted for the 11th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) meeting 2018 include:

Sobi abstracts and Sobi-supported independent research presentation

- Poster P 073: Study design for ReITIrate – A prospective study of rescue ITI with recombinant factor VIII FC (rFVIIIFc) in patients who have failed previous ITI attempts
  Königs C, Meeks S, Jain N, Lethagen S

- Poster P195: Reduced ABR and injection frequency in haemophilia A subjects with short endogenous factor half-life on prophylaxis with rFVIIIFc: A long post hoc analysis
  Winding B, Szamosi J, Lethagen S

- Poster P113: The effect of inhibitors and immune tolerance induction treatment on quality of life for adult patients with severe haemophilia; The CHESS study
  Kritikou P, Noone D, Myren K-J, O’ Hara

- Poster P052: Assessment of clotting activity of recombinant FIX fusion protein in European haemophilia treatment centres
  Willemze A, Sadeghi-Khomami A, Sörskog L, Wikén M, Lethagen S

- Poster P131: Pharmacokinetics simulations of different treatment regimens with rFVIIIFc and rFVIII: Economic impact from a German perspective
  Myren K-J, Gozzi P, Zucca F, Lethagen S

- Poster: How our patients view EHL products in haemophilia expectations expressed before switching (the HOPE study)
  Khair K, O'Driscoll M, Pollard D, Harrison C, Hook S, Holland M, The HOPE study working group

- Sobi-supported Independent Research: Oral presentation OR02: Within-patient comparison of treatment patterns before and after switching to rFVIIIFc: A report from the UK National Haemophilia Database
  Scott MJ, Xiang H, Collins PW, Hay CRM

Abstracts are available through the EAHAD 2018 web site, http://eahadcongress.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. The World Federation of Hemophilia estimates that approximately 180,000 people are currently diagnosed with haemophilia A and B world-wide[i].

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

About Elocta®

Elocta® (efmoroctocog alfa) is a recombinant clotting factor therapy developed for haemophilia A using Fc fusion technology to prolong circulation in the body. 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 (half-life). Elocta is manufactured using a human cell line in an environment free of animal and human additives.

Elocta is approved and marketed by Sobi for the treatment of haemophilia A in the EU, Iceland, Kuwait, Liechtenstein, Norway, Saudi Arabia and Switzerland. It is approved and marketed as ELOCTATE® [Antihemophilic Factor (Recombinant), Fc Fusion Protein] by Bioverativ in the United States, Japan and Canada. It is also approved in Australia, New Zealand, Brazil and other countries, where Bioverativ has the marketing rights.

As with any factor replacement therapy, allergic-type hypersensitivity reactions and development of inhibitors may occur in the treatment of haemophilia A. Inhibitor development has been observed with Elocta, including in previously untreated patients. 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). Alprolix is manufactured using a human cell line in an environment free of animal and human additives.

Alprolix is approved and marketed by Sobi for the treatment of haemophilia B in the EU, Iceland, Kuwait, Liechtenstein, Norway, Saudi Arabia and Switzerland, as well as in the United States, Canada, Japan, Australia, New Zealand, Brazil and other countries where Bioverativ has the marketing rights.

Allergic-type hypersensitivity reactions and development of inhibitors have been observed with Alprolix in the treatment of haemophilia B, including in previously untreated patients. Note that the indication for previously untreated patients is not included in the EU Product Information.

About the Sobi and Bioverativ collaboration

Sobi and Bioverativ collaborate on the development and commercialisation of Alprolix and Elocta/ELOCTATE. Sobi has final development and commercialisation rights in the Sobi territory (essentially Europe, North Africa, Russia and most Middle Eastern markets). Bioverativ has final development and commercialisation rights in North America and all other regions in the world excluding the Sobi territory, and has manufacturing responsibility for Elocta/ELOCTATE and Alprolix. While Fc fusion technology has been used for more than 15 years, Sobi and Bioverativ have optimised the technology and are the first companies to utilise it in the treatment of haemophilia.

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 2016, Sobi had total revenues of SEK 5.2 billion (USD 608 M) and about 760 employees. The share (STO: SOBI) is listed on Nasdaq Stockholm. More information is available at www.sobi.com.

For more information please contact

Media relations
Linda Holmström, Senior Communications Manager
+ 46 708 73 40 95, + 46 8 697 31 74
linda.holmstrom@sobi.com

Investor relations
Jörgen Winroth, Vice President, Head of Investor Relations
+1 347 224 0819, +1 212 579 0506
jorgen.winroth@sobi.com

[i] World Federation of Hemophilia, Annual Global Survey 2015, published in October 2016. Available at: http://www.wfh.org/en/data-collection
[ii] World Federation of Hemophilia. About Bleeding Disorders – Frequently Asked Questions. Available at: http://www.wfh.org/en/page.aspx?pid=637#Difference_A_B. Accessed on: June 17, 2016.
[iii] 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