Extension study data supporting long-term safety and efficacy of Eloctate®/Elocta® published in *Haemophilia*

- Interim results show participants maintained low bleeding rates with prophylactic dosing

Newly published clinical data demonstrate that people on extended-interval prophylaxis regimens with Eloctate®/Elocta® (rFVIIIFc) [Antihemophilic Factor (Recombinant), Fc Fusion Protein] experienced low bleeding rates, Biogen (NASDAQ: BIIB) and Swedish Orphan Biovitrum AB (publ) (Sobi) (STO: SOBI) announced today. The interim results of this phase 3, open-label extension study called ASPIRE were published in the online edition of *Haemophilia*, the journal of the World Federation of Hemophilia, the European Association for Haemophilia and Allied Disorders, and the Hemostasis & Thrombosis Research Society.

Study participants completing the phase 3 A-LONG and Kids A-LONG studies were eligible to participate in ASPIRE. The results to-date show the majority of participants in ASPIRE, maintained or extended their dosing intervals between treatments compared to the A-LONG and Kids A-LONG studies. As of the interim analysis, the median time in the ASPIRE study was 80.9 weeks for adults and adolescents completing the A-LONG study, and 23.9 weeks for children completing the Kids A-LONG study. Inhibitor development is the primary endpoint of ASPIRE and no inhibitors were reported in any treatment groups. Through the interim ASPIRE analysis, adults and adolescents experienced annualised bleeding rates (ABRs) of 0.66, 2.03 and 1.97 in the individualised, weekly and modified prophylaxis arms, respectively. Children on individualised prophylaxis also experienced low bleeding rates, with an overall median ABR of 0.0 in children less than 6 years of age, and 1.54 for children ages 6 to 12. These results were consistent with data from the phase 3 A-LONG and Kids A-LONG studies.

In addition to efficacy and safety endpoints, the publication also reports changes in prophylactic injection frequency from the end of the A-LONG study through the interim analysis. Of the adults and adolescents who had previously been treated prophylactically and who remained in the study through the interim analysis (n=128), 72 percent maintained their prophylactic dosing interval and 22 percent lengthened and six percent shortened the time between injections. Extension study participants could change treatment group at any time.

“The design of the ASPIRE study provides physicians a high degree of dosing flexibility, with the goal of reflecting their real-world treatment practices,” said Guy Young, M.D., Director of the Hemostasis and
Thrombosis Center, Children’s Hospital of Los Angeles. “The results suggest prophylaxis with Eloctate/Elocta shows efficacy and safety for the long-term treatment of haemophilia A.”

In ASPIRE, most participants received prophylactic treatment and were able to maintain protection against bleeding episodes with Eloctate/Elocta consumption that was consistent with that observed in A-LONG and Kids A-LONG.

**Growing body of evidence further validates Eloctate/Elocta clinical profile**

The publication reported cumulative duration of treatment from the beginning of the A-LONG and Kids A-LONG studies through the ASPIRE interim data analysis. The median cumulative duration of treatment was 117.7 weeks for adults and adolescents, and 51.5 weeks for children less than 12 years old.

Across age groups, safety results were consistent with the general haemophilia A population. There were no reports of serious allergic reactions or vascular clots. The most common adverse events (incidence of greater than or equal to five percent) included nasopharyngitis (common cold), arthralgia (joint pain) and upper respiratory infection.

“These published results add to the body of data demonstrating Eloctate’s/Elocta’s safety profile and ability to provide protection against bleeding episodes,” said Wing-Yen Wong, M.D., vice president, Global Medical, Hematology and Immunology at Biogen. “As a company focused on bringing treatment advances to the haemophilia community, we are committed to gathering comprehensive, long-term clinical data across populations.”

“We remain focused on the goal of elevating haemophilia care globally, and we believe the publication of these data is important clinical research that contributes to the advancement of medical science in haemophilia,” said Birgitte Volck, M.D., Ph.D., senior vice president and chief medical officer of Sobi. “These interim extension data help affirm the known efficacy and safety of Eloctate/Elocta for the treatment of people with haemophilia A.”

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**About ASPIRE**

ASPIRE is an open-label, non-randomised, multi-year extension study for people who completed the pivotal, Phase 3 A-LONG or Kids A-LONG studies. The study enrolled 211 males, including 150 (98 per cent) of those who completed A-LONG and 61 (91 per cent) of those who completed Kids A-LONG. The primary endpoint is the development of inhibitors. Secondary endpoints include the annualised number of bleeding episodes per subject, Eloctate/Elocta exposure days and a participant’s assessment of response to treatment of a bleeding episode.
About Eloctate/Elocta (rFVIIIFc)

rFVIIIFc is a long-acting recombinant factor VIII Fc fusion protein product candidate for people with haemophilia A. Eloctate [Antihemophilic Factor (Recombinant), Fc Fusion Protein], is the first recombinant clotting factor VIII therapy with prolonged circulation in the body for adults and children with haemophilia A approved in the United States, Canada, Australia and Japan. Elocta was submitted to the European Medicines Agency (EMA) for regulatory approval in Europe in October 2014. Eloctate/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). It is believed that this enables Eloctate/Elocta to use a naturally occurring pathway to prolong the time the therapy remains in the body. While Fc fusion has been used for more than 15 years, Biogen is the only company to apply it to the treatment of haemophilia.

About haemophilia A

Haemophilia A is a rare, chronic, genetic disorder in which the ability of a person’s blood to clot is impaired, due to missing or reduced levels of a protein known as factor VIII. People with haemophilia A experience recurrent and extended bleeding episodes that cause pain and irreversible joint damage. Some of these bleeding episodes can be life-threatening. According to the World Federation of Hemophilia, an estimated 142,000 people worldwide are identified living with haemophilia A. Prophylactic injections of factor VIII can temporarily replace the clotting factor necessary to control bleeding and prevent new bleeding episodes.

Inhibitor development is a response of the body’s immune system that interferes with the activity of therapy. About 25 to 30 per cent of people with severe haemophilia A develop inhibitors during their lifetime. Inhibitors typically develop after a median of 8-10 exposure days, though this number varies widely.

About the Sobi and Biogen collaboration

Sobi and Biogen are collaboration partners in the development and commercialisation of Elocta/Eloctate/rFVIIIFc for haemophilia A. Sobi has final development and commercialisation rights for Elocta/rFVIIIFc in the Sobi territories (Europe, North Africa, Russia and certain Middle Eastern markets). Biogen leads development for Elocta/Eloctate/rFVIIIFc, has manufacturing rights, and has commercialisation rights in North America and all other regions in the world excluding the Sobi territories.

About Biogen

Through cutting-edge science and medicine, Biogen discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hematologic conditions and autoimmune disorders. 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 haemophilia therapies. For product labelling, press releases and additional information about the company, please visit www.biogen.com.

About Sobi

Sobi is an international specialty healthcare company dedicated to rare diseases. Our 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. We also market 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.
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