PRESS RELEASE

Stockholm, Sweden, 10 December 2017

Study shows weekly prophylactic treatment with Elocta® resulted in bleed protection and target joint resolution in people with haemophilia A

Swedish Orphan Biovitrum AB (publ) (Sobi™)(STO:SOBI) and Bioverativ Inc. (NASDAQ: BIVV) today announce the results of a new, post-hoc, longitudinal analysis of the pivotal Phase 3 A-LONG study and ASPIRE long-term extension study, showing that weekly prophylactic dosing with its extended half-life therapy Elocta® (efmoroctocog alfa) marketed as ELOCTATE® [Antihemophilic Factor (Recombinant), Fc Fusion Protein] in the US, has the potential to provide improved bleed protection over episodic treatment, resolve target joints and reduce the treatment burden associated with more frequent dosing intervals. The analysis is being presented today in a poster session at the 59th Annual Meeting of the American Society of Hematology.

Elocta was developed using Fc fusion technology to prolong circulation in the body and its efficacy and safety have been studied in haemophilia A patients since 2010. This new, post-hoc analysis supports a growing body of clinical data showing prophylactic treatment with Elocta can positively impact long-term joint health. Elocta is currently not indicated for weekly dosing.

“One of the challenges for people with severe haemophilia A can be treatment every few days with inadequate bleed protection,” said Maha Radhakrishnan, M.D., Senior Vice President of Medical at Bioverativ. “We are committed to improving patient outcomes and continue to explore how ELOCTATE can meaningfully make a difference for patients with the potential for longer dosing intervals that could provide continued joint health improvement.”

Prophylactic treatment with factor therapy is recognized as the optimal therapy for severe haemophilia A, yet, according to the World Federation of Hemophilia guidelines, this treatment regimen traditionally involves injections three times per week with conventional factor based products. With Elocta’s extended half-life, patients can extend dosing intervals up to five days resulting in less frequent injections. Using data spanning four years from the pivotal Phase 3 A-LONG study, and ASPIRE, the long-term extension study, researchers examined subjects who were exposed to a seven-day dosing (65 IU/kg/wk) interval to assess long-term outcomes as determined by annualized bleeding rates (ABR), adherence and resolution of target joints.

In the study, 43 adults and adolescents (>12 years) were exposed to an Elocta weekly dosing interval for a median study duration of 3.1 years. Researchers also analysed results of those who maintained a weekly dosing interval throughout the study period (n=19).

For those subjects in the ever-on-weekly dosing group who had pre-study episodic treatment (n=32), transition to weekly prophylaxis dosing resulted in a change in median ABR (IQR) of -23.7 (-35.8, -12.8). For those subjects who were always on a weekly dosing regimen throughout the study period (n=19), the median pre-study ABR (IQR) for subjects on a pre-study episodic regimen was 29 (18, 45) compared to an on-study ABR (IQR) of 1.7 (0.5, 6.7). Subjects experienced protection from spontaneous bleeds (median spontaneous ABR (IQR) of 1.2 (0.2, 2.8) for subjects ever-on-weekly dosing and 0.7 (0, 1.6) for subjects always-on-weekly-dosing and from spontaneous joint bleeds (median ABR (IQR) of 0.8 (0, 2.5) in subjects ever-on-weekly dosing and 0.2 (0, 1.0) in subjects always-on-weekly-dosing).

All subjects were highly adherent while on the weekly dosing regimen (median duration of 3.1 years) and among subjects who chose to initiate a weekly dosing regimen on Elocta at any point of the study, the majority stayed on weekly dosing. One hundred percent of all evaluable target joints in both the ever-on-weekly dosing group and always-on-weekly dosing group resolved during the study period. Study findings suggest weekly dosing may be a reasonable prophylaxis regimen for patients receiving episodic treatment, who would prefer the benefit of prophylaxis and better bleed protection, but with minimal treatment burden.

“Together with Bioverativ, we have long been committed to transforming the care of people with haemophilia through our treatments and ongoing research,” said Armin Reininger, M.D., Ph.D., Head of Medical and Scientific Affairs, Sobi. “These data show the potential of Elocta to make a difference for patients, to be able to extend their dosing intervals based on their needs, with improved joint health, and the possibility to reduce the burden of chronic treatment in patients with haemophilia.”

About ASPIRE

ASPIRE is an open-label, non-randomized, 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 percent) of those who completed A-LONG and 61 (91 percent) of those who completed Kids A-LONG. The primary endpoint is the development of inhibitors. Secondary endpoints include the annualized number of bleeding episodes per subject, Elocta® exposure days and a participant’s assessment of response to treatment of a bleeding episode.

About haemophilia A

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. The World Federation of Hemophilia estimates that approximately 150,000 people are currently diagnosed with haemophilia A world-wide[i].

People with haemophilia A experience bleeding episodes that can cause pain, irreversible joint damage and life-threatening haemorrhages. Prophylactic injections of factor VIII 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. 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. Elocta is manufactured using a human cell line in 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, Kuwait and the Kingdom of Saudi Arabia, marketed by Sobi. It is approved and marketed as ELOCTATE® by Bioverativ in the United States, Japan and Canada. It is also approved in Australia, New Zealand, Brazil and other countries, and Bioverativ has 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 haemophilia A. Inhibitor development has been observed with Elocta/ELOCTATE including in previously untreated patients. For more information, please see the full U.S. prescribing information. Note that the indication for previously untreated patients is not included in the EU Product Information.

About Bioverativ

Bioverativ is a global biopharmaceutical company dedicated to transforming the lives of people with hemophilia and other rare blood disorders through world-class research, development and commercialization of innovative therapies. Launched in 2017 following separation from Biogen Inc., Bioverativ builds upon a strong heritage of scientific innovation and is committed to actively working with the blood disorders community. The company's mission is to create progress for patients where they need it most and its hemophilia therapies when launched represented the first major advancements in hemophilia treatment in more than two decades. For more information, visit www.bioverativ.com or follow @bioverativ 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 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.

About the Bioverativ and Sobi collaboration

Sobi and Bioverativ 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). 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.

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[i] World Federation of Hemophilia, Annual Global Survey 2016, published in October 2017. 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