Study Shows Weekly Prophylactic Treatment with ELOCTATE® Resulted in Bleed Protection and Target Joint Resolution in People with Hemophilia A

ATLANTA– December 10, 2017 – Bioverativ Inc. (NASDAQ: BIVV) and Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) 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 ELOCTATE® [Antihemophilic Factor (Recombinant), Fc Fusion Protein], marketed as Elocta® in Europe and the Middle East, 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.

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

“One of the challenges for people with severe hemophilia 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 hemophilia 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 ELOCTATE’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 interval (65 IU/kg/wk) 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 ELOCTATE weekly dosing interval for a median study duration of 3.1 years. Researchers also analyzed 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 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 ELOCTATE at any point during 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 hemophilia 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 ELOCTATE 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 hemophilia.”

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, ELOCTATE exposure days and a participant’s assessment of response to treatment of a bleeding episode.

About ELOCTATE®/Elocta®
ELOCTATE® [Antihemophilic Factor (Recombinant), Fc Fusion Protein] is a recombinant clotting factor therapy developed for hemophilia 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 ELOCTATE 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, Bioverativ and Swedish Orphan Biovitrum AB (publ) (Sobi) have optimized the technology and are the
first companies to utilize it in the treatment of hemophilia. ELOCTATE is manufactured using a human cell line in an environment free of animal and human additives.

ELOCTATE is approved and marketed 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. It is also approved as Elocta® in the European Union, Switzerland, Iceland, Liechtenstein, Norway and other countries where it is marketed by Sobi.

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 Hemophilia A**

Hemophilia is a rare, genetic disorder in which the ability of a person's blood to clot is impaired. Hemophilia 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 hemophilia A worldwide.

People with hemophilia A experience bleeding episodes that can cause pain, irreversible joint damage and life-threatening hemorrhages. Prophylactic injections of factor VIII can temporarily replace the clotting factor that is needed to control bleeding and prevent new bleeding episodes. The World Federation of Hemophilia (WFH) recommends prophylaxis as the optimal therapy as it can prevent bleedings and joint destruction.

**About Bioverativ**

Bioverativ (NASDAQ: BIVV) 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](http://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 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 Bioverativ and Sobi Collaboration**

Bioverativ and Sobi collaborate on the development and commercialization of ALPROLIX and ELOCTATE® [Antihemophilic Factor (Recombinant), Fc Fusion Protein], which is marketed as Elocta® in Europe. Bioverativ has final development and commercialization rights in North America and all other regions in the world excluding the Sobi territory, and has manufacturing responsibility for ELOCTATE and ALPROLIX. Sobi has final development and commercialization rights in the Sobi territory (essentially Europe, North Africa, Russia and most Middle Eastern markets).
Bioverativ Safe Harbor
This press release contains forward-looking statements, including statements about the potential benefits of ELOCTATE in hemophilia A patients, including with weekly prophylactic dosing. These forward-looking statements may be accompanied by such words as “anticipate,” “believe,” “could,” “estimate,” “expect,” “forecast,” “intend,” “may,” “plan,” “potential,” “project,” “target,” “will” and other words and terms of similar meaning. You should not place undue reliance on these statements. These statements involve risks and uncertainties that could cause Bioverativ’s actual results to differ materially from those reflected in such statements, including, without limitation, risks related to development of early stage programs, unexpected concerns that may arise from additional data or analysis, regulatory authorities may require additional information or further studies, regulatory authorities may fail to expand product labeling, and other risks and uncertainties associated with Bioverativ’s drug development and commercialization activities described in the Risk Factors section of Bioverativ’s filings with the Securities and Exchange Commission. These statements are based on Bioverativ’s current beliefs and expectations and speak only as of the date of this press release. Bioverativ does not undertake any obligation to publicly update any forward-looking statements.

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