New Data Show Extended Prophylactic Dosing with ALPROLIX® Provides Safe and Effective Protection in People with Severe Hemophilia B

- Post-hoc longitudinal analysis from B-LONG and B-YOND studies shows patients who progressed to individualized prophylactic dosing intervals of 14 days or longer maintained low annualized bleeding rates

ATLANTA – December 11, 2017 – Bioverativ Inc. (NASDAQ: BIVV) and Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) today announced the results of a new, post-hoc longitudinal analysis demonstrating that individualized dosing with extended half-life therapy, ALPROLIX® [Coagulation Factor IX (Recombinant), Fc Fusion Protein], every 14 or more days may be a potential option for people with severe hemophilia B who seek the benefits of protection from a prophylactic therapy with reduced treatment burden. The analysis is being presented in a poster session at the 59th Annual Meeting of the American Society of Hematology (ASH).

ALPROLIX is a recombinant clotting factor IX therapy developed using Fc fusion technology to prolong circulation in the body. ALPROLIX has the longest real-world experience of any hemophilia B extended half-life therapy and has been studied in more than 150 adult, adolescent, and pediatric patients over three years as part of a robust clinical development program and an extension study.

Using data from the pivotal Phase 3 B-LONG study of 123 patients with severe hemophilia B, and B-YOND, the long-term extension study of ALPROLIX that included 93 patients from B-LONG, researchers evaluated long-term outcomes of 22 study participants (adults and adolescents ≥12 years) in the individualized treatment group who progressed to long-term prophylactic dosing regimens of 14 days or longer. Data from this longitudinal analysis showed these study participants achieved consistent bleed protection with extended prophylactic dosing intervals for up to three years.

“These data show that individualized prophylactic treatment with ALPROLIX, starting at weekly or ten-day dosing intervals with the possibility to extend to 14 days or longer, has the potential to deliver optimal protection against bleeds for people with hemophilia B,” said Maha Radhakrishnan, M.D., Senior Vice President of Medical at Bioverativ. “Individualized dosing intervals allow patients and their physicians to personalize treatment plans that balance the need to maximize bleed protection while minimizing treatment burden. We remain committed to improving the long-term outcomes for people with hemophilia B.”

Data for 22 study participants on varying pre-study treatment regimens, including those who switched to extended dosing at any time during the study, were included in this longitudinal review. Prior to treatment
with ALPROLIX, 10 of the 22 study participants had received prophylactic treatment and 12 participants were on episodic treatment. Results from this analysis showed that:

- Patients who received pre-study prophylactic treatment were well protected with extended dosing intervals of 14 days or longer with an annualized bleed rate (ABR) of 1.8 as compared to 2.0 pre-study.
- Median ABR decreased from 25 (22 - 36) to 1.4 (0.6–5.8) for the participants who received pre-study episodic treatment.
- The median (IQR) duration of treatment on the ≥14-day regimen in the 22 patients was 3.4 years (1.8-4.0).
- Study participants treated with ≥14 dosing intervals were well controlled with a median spontaneous ABR of 0.7 over three years.

“These findings reinforce a history of successfully delivering long-acting protection against spontaneous and joint bleeds in hemophilia B by dosing with ALPROLIX at one to two-week intervals,” said Armin Reininger, M.D., Ph.D., Head of Medical and Scientific Affairs, Sobi. “In collaboration with Bioverativ, we will continue to explore the potential of ALPROLIX to reduce the burden of disease and create meaningful improvement in the lives of people living with hemophilia.”

Earlier at ASH, Bioverativ presented data from the preclinical imaging study, *Extravascular Distribution of Conventional and EHL FIX Products Using In Vivo SPECT Imaging Analysis in Hemophilia B Mice* (link), which showed that ALPROLIX demonstrates higher tissue distribution and retention in joint areas compared to other factor IX molecules. These results were part of an ongoing imaging collaboration with Invicro, LLC, to investigate the impact of extravascular distribution of factor IX therapies, including ALPROLIX, on protection from bleeds and improvement in joint health.

**About the B-YOND Extension Study**

B-YOND enrolled 116 previously-treated males, including 93 participants (81%) who completed B-LONG, and 23 (100%) of those who completed Kids B-LONG. The primary outcome measure is development of inhibitors. Secondary endpoints include the annualized number of bleeding episodes per subject (including spontaneous joint bleeding rates), ALPROLIX exposure days per participant, ALPROLIX consumption (total IU/kg per subject per year), and the participant’s assessment of response to treatment of a bleeding episode.

**About ALPROLIX®**

ALPROLIX® [Coagulation Factor IX (Recombinant), Fc Fusion Protein] is a recombinant clotting factor therapy developed for hemophilia 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, Bioverativ and Sobi have optimized the technology and are the first companies to utilize it in the treatment of hemophilia. ALPROLIX is manufactured using a human cell line in an environment free of animal and human additives.

ALPROLIX is approved and marketed by Bioverativ for the treatment of hemophilia B 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 authorized in the European Union, Iceland, Liechtenstein, Norway, Switzerland, Kuwait and Saudi Arabia where it is marketed by Sobi.

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 Hemophilia B
Hemophilia B is caused by having substantially reduced or no factor IX activity in the blood, which is needed for normal clotting.¹ The World Federation of Hemophilia estimates that approximately 29,700 people are currently diagnosed with hemophilia B worldwide.²

People with hemophilia B may experience bleeding episodes in joints and muscles that cause pain, decreased mobility and irreversible joint damage. In the worst cases, these bleeding episodes can cause organ bleeds and life-threatening hemorrhages. Infusions of factor IX temporarily replace clotting factors necessary to resolve bleeding and, when used prophylactically, to prevent new bleeding episodes.¹

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 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
Bioverativ and Sobi collaborate on the development and commercialization of ALPROLIX® [Coagulation Factor IX (Recombinant), Fc Fusion Protein] 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 individualized prophylactic treatment with ALPROLIX in patients with hemophilia B. 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, unexpected concerns that may arise from data, findings, analysis or results obtained from research or clinical trials or post hoc analysis of studies, 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.

BIOVERATIV

Media Contact: Marianne McMorrow
+1 781 663 4376
media@bioverativ.com

Investor Relations Contact: Susan Altschuller
+1 781 663 4360
IR@bioverativ.com

SOBI

Media Contact: Linda Holmström
+ 46 708 73 40 95
linda.holmstrom@sobi.com

Investor Relations Contact: Jörgen Winroth
+1 347-224-0819
jorgen.winroth@sobi.com

References
