ALPROLIX® Demonstrates Higher Tissue Distribution and Retention in Joints Compared to Other Factor IX Molecules in a Preclinical Imaging Study

ATLANTA – December 9, 2017 – Bioverativ Inc. (NASDAQ: BIVV), a global biopharmaceutical company dedicated to transforming the lives of people with rare blood disorders, today announced findings from a novel imaging study investigating extravascular distribution of factor IX therapies, including its leading extended half-life therapy, ALPROLIX® [Antihemophilic Factor IX (Recombinant), Fc Fusion Protein]. The preclinical study showed ALPROLIX had a higher level of extravascular distribution and retention in certain joint areas when compared with conventional factor IX and a glycoPEGylated factor IX analog. The study was conducted in collaboration with Invicro, LLC, a leading imaging services provider, and is being presented today at the 59th Annual Meeting of the American Society of Hematology.

Joint disease, which is caused by frequent bleeding into joints over time, is one of the most common complications for people with hemophilia and often results in chronic pain and significant disability. The ability to prevent or effectively treat joint bleeds is crucial to reduce the risk of joint damage over the long term.

“It is becoming evident that the modification used to extend half-life may influence the way factor IX therapies are distributed in the body and their potential therapeutic benefit for patients,” said Rob Peters, Ph.D., Senior Vice President of Research at Bioverativ. “This preclinical study shows that ALPROLIX consistently delivered more factor to certain critical joint areas. We are committed to raising the standard of care for people with hemophilia B, and we intend to explore the impact of factor distribution in the joints further through ongoing studies with Invicro.”

In this study, radiolabeled ALPROLIX, conventional factor IX therapy (rFIX) and a glycoPEGylated factor IX (GP-rFIX) analog were administered in a preclinical model of hemophilia B and imaged at a total of five time points. In vivo SPECT imaging revealed ALPROLIX showed significantly higher distribution to extravascular areas such as knee and shoulder joints throughout all time points measured (up to five half-lives post dosing) when compared to conventional factor IX therapy (rFIX) and the glycoPEGylated factor IX (GP-rFIX) analog, even when factor IX plasma levels were below detectable levels. Of the three therapies evaluated, the glycoPEGylated factor IX (GP-rFIX) analog showed the lowest distribution to joint areas. Ongoing studies are aimed at further characterizing the extravascular distribution of factor therapy using innovative means of imaging.
Bioverativ and Invicro entered into a strategic imaging collaboration to study the importance of factor IX distribution to tissue and its role in improving joint health. The companies will also work to develop standardized protocols to monitor and assess joint health using ultrasound imaging in people with hemophilia in research studies of their marketed and pipeline products and in clinical practice.

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 its collaboration partner, Swedish Orphan Biovitrum AB (publ) (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.

Bioverativ Safe Harbor
This press release contains forward-looking statements, including statements about the potential benefits of ALPROLIX based on observations in preclinical studies, and potential plans, advancements, developments and benefits that may be achieved or derived from the collaboration with Invicro. These statements may be identified by words such as "believe," "expect," "may," "plan," "potential," "will" and similar expressions, and are based on Bioverativ’s current beliefs and expectations. You should not place undue reliance on these statements. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including, without limitation, risk and uncertainties regarding early stage research and trials, which may not be indicative of full results or results from later stages of clinical trials; unexpected concerns may arise from data, analysis or results obtained during 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; uncertainty regarding the ability to achieve the expected benefits from the collaboration; reliance on third parties over which Bioverativ may not always have full control; and other risks and uncertainties that are described in the Risk Factors section of Bioverativ’s most recent annual or quarterly report filed with the Securities and Exchange Commission. Any forward-looking statements speak only as of the date of this press release and Bioverativ assumes no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

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