

PRESS RELEASE

Bioverativ Announces FDA Acceptance of Investigational New Drug Application for BIVV001 to Treat Hemophilia A

- *BIVV001 is a novel, investigational factor VIII therapy designed to extend protection from bleeds with prophylaxis dosing of once weekly or longer*
- *On track to initiate a Phase 1/2a clinical trial in the second half of 2017*

Waltham, Mass. – June 12, 2017 – [Bioverativ](#) (NASDAQ: BIVV), a global biotechnology company focused on the discovery, development and commercialization of innovative therapies for hemophilia and other rare blood disorders, today announced that the U.S. Food and Drug Administration (FDA) has accepted the company's Investigational New Drug (IND) application for BIVV001 (also known as rFVIII-Fc-VWF-XTEN), a novel, investigational factor VIII therapy designed to potentially extend protection from bleeds with prophylaxis dosing of once weekly or longer for people with hemophilia A.

"People with hemophilia A continue to have a strong unmet need for therapies that can provide protection from bleeds and favorable long-term outcomes in terms of joint health and quality of life with once weekly dosing," said Tim Harris, Ph.D., D.Sc., executive vice president of research and development at Bioverativ. "In 2014, we made significant progress toward this goal with the introduction of ELOCTATE®, the leading extended half-life therapy for hemophilia A. This IND acceptance expands on that legacy and reflects our continued commitment to advancing scientific innovation that can make a meaningful difference for people with hemophilia."

BIVV001 builds on the company's existing Fc fusion technology by adding a region of von Willebrand factor and XTEN polypeptides to potentially extend its time in circulation. It is the only investigational factor VIII therapy in development that is designed to overcome the von Willebrand factor ceiling, which is believed to impose a half-life limitation on current factor VIII therapies.

"BIVV001 is the first molecule of its kind to fuse four different proteins together to address the challenges of hemophilia A," said Rob Peters, Ph.D., senior vice president of research at Bioverativ. "We are encouraged by our extensive preclinical data which show improved pharmacokinetics that are independent of von Willebrand factor, and look forward to commencing our Phase 1/2a clinical trial later this year."

About Hemophilia A

Hemophilia 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 hemophilia A experience bleeding episodes that may cause pain, irreversible joint damage and life-threatening hemorrhagesⁱ. The World Federation of Hemophilia recommends prophylaxis as the optimal therapy as it can prevent bleedings and joint destruction and may improve quality of lifeⁱⁱ. An estimated 150,000 people worldwide are identified as living with hemophilia Aⁱⁱⁱ.

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 and 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 Bioverativ

Bioverativ is a global biotechnology 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](https://twitter.com/bioverativ) on Twitter.

Bioverativ Safe Harbor

This press release contains forward-looking statements, including statements about the potential benefits, safety and clinical effects of BIVV001, and expected timing of clinical trials. 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. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. Factors which could cause actual results to differ materially from Bioverativ's current expectations include uncertainties relating to the initiation, enrollment and completion of stages of clinical trials, unexpected concerns may arise from data, analysis or results obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of product candidates, or Bioverativ may encounter other unexpected hurdles. For more detailed information on the risks and uncertainties associated with Bioverativ's drug development and commercialization activities, please review 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.

MEDIA CONTACT:

Marianne McMorrow
+1 781 663-4376
media@bioverativ.com

INVESTOR CONTACT:

Susan Altschuller
+1 781 663-4360
IR@bioverativ.com

ⁱ 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 May 2017

ⁱⁱ Guideline for the management of hemophilia, World Federation of Hemophilia, 2nd edition, <https://www1.wfh.org/publication/files/pdf-1472.pdf>. Accessed on December 2015

ⁱⁱⁱ World Federation of Hemophilia, Annual Global Survey 2015, published in October 2016. Available at: <https://www.wfh.org/en/data-collection>