



The New England Journal of Medicine Publishes Pivotal Data Demonstrating Prophylactic Infusions of ALPROLIX™ Effectively Controlled Bleeding in Hemophilia B Patients

December 4, 2013

– B-LONG Study Showed Investigational Therapy Administered Once Every One to Two Weeks Prevented or Reduced Bleeding Episodes –

CAMBRIDGE, Mass. & STOCKHOLM--([BUSINESS WIRE](#))--Today [Biogen Idec](#) (NASDAQ: BII) and [Swedish Orphan Biovitrum](#) AB (publ) (Sobi) (STO: SOBI) announced the publication of detailed results from the pivotal Phase 3 study of ALPROLIX™, the companies' investigational long-lasting recombinant factor IX Fc fusion protein candidate for hemophilia B. The study appears in the Online First edition and will appear in the December 12 print issue of *The New England Journal of Medicine* (NEJM).

The study of ALPROLIX showed that people with severe hemophilia B safely and effectively prevented or reduced bleeding episodes with prophylactic infusions every one to two weeks. As the first long-lasting investigational therapy for hemophilia B to complete a Phase 3 study, ALPROLIX has the potential to be the first important advance in hemophilia B treatment in more than 16 years.

The study, called B-LONG, is the largest Phase 3 clinical study in hemophilia B ever completed. It examined the effect of ALPROLIX therapy delivered with multiple dosing regimens, including prophylactic (weekly or longer), episodic (on-demand) and surgical (perioperative management). Results of B-LONG formed the basis of regulatory applications for ALPROLIX, which are currently under review in several countries including the United States, Canada, Australia and Japan.

"Today, many people with hemophilia B do not follow a prophylactic regimen, and the burdensome infusion schedules associated with currently available treatment may contribute to its limited adoption," said Marilyn Manco-Johnson, M.D., professor of Pediatrics, University of Colorado and director, Hemophilia and Thrombosis Center, University of Colorado and Children's Hospital, Colorado. "The National Hemophilia Foundation recommends a prophylactic regimen as optimal for people with severe hemophilia, and studies show this approach reduces bleeding episodes and associated risks. It is my hope that long-lasting therapies in development, such as ALPROLIX, may lessen the burden of prophylactic dosing for people with hemophilia B, and encourage adoption."

[Hemophilia B](#) is a rare, chronic, inherited disorder in which the ability of a person's blood to clot is impaired, which can lead to extended bleeding episodes. Currently available therapy requires prophylactic infusions two times a week or more. Frequent prophylactic infusions can be a burden to people with hemophilia and may reduce adoption to this type of treatment regimen.¹ ALPROLIX has a prolonged circulation time in the blood, and utilizes a technology called Fc fusion. The B-LONG study showed that the interval between prophylactic infusions was extended with ALPROLIX, so that infusions were only needed once a week to once every two weeks in the study. If approved, ALPROLIX may enable people with severe hemophilia B to receive fewer prophylactic infusions than currently available therapy.

"Many of us at Biogen Idec have personal connections to the hemophilia community and know firsthand the burden of frequent infusions," said Glenn Pierce, M.D., Ph.D., senior vice president of Global Medical Affairs and chief medical officer at Biogen Idec's hemophilia therapeutic area. "If approved, long-lasting ALPROLIX therapy will have the potential to change the way hemophilia B is managed and address a critical need for patients."

B-LONG Study Results

B-LONG was a global, open-label, multi-center Phase 3 study that evaluated the efficacy, safety and pharmacokinetics (measurement of the presence of the drug in a patient's body over time) of ALPROLIX in 123 males aged 12 years and older with hemophilia. The study involved 50 hemophilia treatment centers in 17 countries on six continents.

The B-LONG study evaluated ALPROLIX via four treatment regimens:

- Weekly prophylaxis (arm 1)
- Individualized-interval prophylaxis dosing – starting at every 10 days (arm 2)
- Episodic (on-demand) therapy as needed to manage bleeding episodes (arm 3)
- Perioperative (surgical) management (arm 4)

Dose (arm 1) and interval (arm 2) were adjusted during the study to maintain target factor IX levels intended to prevent bleeding.

The overall median annualized bleeding rates (ABR), or projected rate of bleeding episodes per year, were 3.0 for weekly prophylaxis arm and 1.4 for individualized-interval prophylactic regimens, compared to 17.7 for the episodic therapy group. The median dosing interval with individualized-interval prophylaxis (arm 2) was 12.5 days. Bleeding episodes for participants in arms 1-3 were documented and more than 90 percent of all bleeding episodes were controlled by a single infusion of ALPROLIX. ALPROLIX was assessed in the perioperative management of 12 study participants undergoing 14 major surgical procedures. The treating physicians rated response to surgery of ALPROLIX as "excellent" or "good" in 100 percent of these surgeries.

No participants in the study developed inhibitors to ALPROLIX (antibodies that may interfere with the activity of the therapy). There were no reports of vascular clots or serious allergic reactions. Overall, safety events were consistent with those expected in the general hemophilia population. The most common adverse events (incidence of ≥5 percent) occurring outside of the perioperative period were nasopharyngitis (common cold), influenza (flu), arthralgia (joint pain), upper respiratory infection, hypertension (high blood pressure) and headache. One participant had a single serious adverse event that was considered to be possibly related to treatment with ALPROLIX. The participant, who had a history of hematuria (presence of blood in the urine), developed an obstructive clot in the urinary collecting system; he continued ALPROLIX treatment and the event resolved with medical management.

"The publication of the B-LONG pivotal study in *The New England Journal of Medicine* is a significant milestone that will contribute to the advancement of medical science in hemophilia care," said Birgitte Volck, M.D., Ph.D., senior vice president development and chief medical officer of Sobi. "These data support the potential of ALPROLIX to provide a meaningful new option to people with hemophilia B by addressing the need for long-lasting therapies for this population."

About ALPROLIX

ALPROLIX is an investigational fully recombinant, long-lasting clotting factor therapy being developed for hemophilia B. It uses Fc fusion technology, which takes advantage of a naturally occurring pathway that delays the breakdown of Immunoglobulin G Subclass 1, or IgG1 (protein commonly found in the body), and cycles it back into the bloodstream. The Fc portion of IgG1 is fused to factor IX in ALPROLIX and is thought to be responsible for the prolonged time ALPROLIX circulates in the body. While Fc fusion is an established technology that has been used for more than 15 years, Biogen Idec is the only company to apply it in hemophilia.

About Hemophilia B

Hemophilia B is a rare, inherited disorder in which the ability of a person's blood to clot is impaired. Hemophilia B occurs in about one in 25,000 male births annually, and more rarely in females, affecting about 3,300 people in the United States. The World Federation of Hemophilia global survey conducted in 2011 estimates that more than 25,000 people are currently diagnosed with hemophilia B worldwide. It is caused by having substantially reduced or no factor IX activity, which is needed for normal blood clotting. People with hemophilia B experience prolonged bleeding episodes that can cause pain, irreversible joint damage and life-threatening hemorrhages. Prophylactic infusions of factor IX can temporarily replace the missing clotting factors that are needed to control bleeding and prevent new bleeding episodes. The Medical and Scientific Advisory Council of the National Hemophilia Foundation recommends prophylaxis as the optimal therapy for people with severe hemophilia B.

About the Biogen Idec and Sobi Collaboration

Biogen Idec and Swedish Orphan Biovitrum (Sobi) are partners in the development and commercialization of ALPROLIX for hemophilia B. Biogen Idec leads development, has manufacturing rights, and has commercialization rights in North America and all other regions excluding the Sobi territory. Sobi has the right to opt in to assume final development and commercialization in Europe (including Russia), the Middle East and Northern Africa.

About Biogen Idec

Through cutting-edge science and medicine, Biogen Idec discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hemophilia and autoimmune disorders. Founded in 1978, Biogen Idec is the world's oldest independent biotechnology company. Patients worldwide benefit from its leading multiple sclerosis therapies, and the company generates more than \$5 billion in annual revenues. For product labeling, press releases and additional information about the company, please visit www.biogenidec.com.

About Sobi

Sobi is an international specialty healthcare company dedicated to rare diseases. Our mission is to develop and deliver innovative therapies and services to improve the lives of patients. The product portfolio is primarily focused on inflammation and genetic diseases, with three late stage biological development projects within hemophilia and neonatology. We also market a portfolio of specialty and rare disease products for partner companies. Sobi is a pioneer in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2012, Sobi had total revenues of SEK 1.9 billion (€ 215 M) and about 500 employees. The share (STO: SOBI) is listed on NASDAQ OMX Stockholm. More information is available at www.sobi.com.

Biogen Idec Safe Harbor

This press release contains forward-looking statements, including statements about the potential advances, impact and therapeutic effect of ALPROLIX, our investigational long-lasting recombinant factor IX candidate. These statements may be identified by words such as "believe," "expect," "may," "plan," "potential," "will" and similar expressions, and are based on our current beliefs and expectations. Drug development and commercialization involve a high degree of risk. Factors which could cause actual results to differ materially from our current expectations include the risk that unexpected concerns may arise from additional data or analysis, regulatory authorities may require additional data or information or further studies, or may fail to approve or may delay approval of our drug candidates, or we may encounter other unexpected hurdles. For more detailed information on the risks and uncertainties associated with our drug development and commercialization activities, please review the Risk Factors section of our 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 we assume no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

¹ Hacker MR, Geraghty S, Manco-Johnson M. Barriers to compliance with prophylaxis therapy in haemophilia. *Haemophilia* : the official journal of the World Federation of Hemophilia 2001;7:392-6.

Multimedia Files:

[Download All Files](#)

 [Preview image](#)

Download:

[Download Thumbnail](#) (2.86 KB)

[Download Web Ready](#) (34.97 KB)

[Download High Resolution](#) (82.32 KB)

[Download ViewImage](#) (7.64 KB)

[Download Square](#) (7.8 KB)

[Download Square](#) (7.8 KB)

Contact:

Biogen Idec

Media Contact:

Todd Cooper, +1 781 464 3260

public.affairs@biogenidec.com

or

Investor Relations Contact:

Ben Strain, +1 781 464 2442

IR@biogenidec.com

or

Swedish Orphan Biovitrum

Media Contact:

Oskar Bosson, +46 70 410 71 80

Head of Communications

or

Analyst/Investor Contact:

Jörgen Winroth, +46 8 697 2135

Vice President, Head of Investor Relations