



## Biogen Idec and Sobi Announce Positive Top-Line Efficacy and Safety Results from Phase 3 ALPROLIX® Pediatric Study

February 27, 2015

*- Study Meets Primary Endpoint and Achieves Low Bleeding Rates with Once-Weekly Prophylactic Dosing in Children -*

*- Results Support EU Regulatory Submission and Future Pediatric Indication Applications -*

CAMBRIDGE, Mass. & STOCKHOLM--([BUSINESS WIRE](#))--[Biogen Idec](#) (NASDAQ: BIIB) and [Swedish Orphan Biovitrum AB](#) (publ) (Sobi) (STO: SOBI) today announced positive top-line results of the Kids B-LONG Phase 3 clinical study that evaluated the safety, efficacy and pharmacokinetics of ALPROLIX® [Coagulation Factor IX (Recombinant), Fc Fusion Protein] in children under age 12 with severe hemophilia B. ALPROLIX was generally well tolerated and no inhibitors (neutralizing antibodies that may interfere with the activity of the therapy) were detected during the study. In this study, once-weekly prophylactic dosing with ALPROLIX resulted in low bleeding rates. ALPROLIX is the only approved hemophilia B therapy with prolonged circulation in the body.

The successful completion of Kids B-LONG supports applications for pediatric indications in several geographies and is an important step in seeking marketing authorization for ALPROLIX in Europe. The European Medicines Agency requires the inclusion of pediatric study data in the initial marketing application for a new hemophilia therapy. Interim results of the Kids B-LONG study helped support the U.S. approval of ALPROLIX for use in children.

"According to published studies, prophylactic treatment for children with severe hemophilia is recommended because it is associated with proven clinical benefits. However, frequent administration schedules can be burdensome for children and their caregivers," said Aoife Brennan, M.D., vice president of hematology clinical development at Biogen Idec. "These data will enable regulatory filings in Europe later this year as well as support pediatric indications in other countries, with the potential to help address a critical need among children with hemophilia B."

Kids B-LONG investigated the safety, efficacy, and pharmacokinetics (measurement of the presence of the drug in a person's body over time) of ALPROLIX in previously treated children under age 12 with severe hemophilia B. The study's primary endpoint was to evaluate the occurrence of inhibitor development. Secondary endpoints included the overall and spontaneous annualized bleeding rates (ABR), which is the estimated number of yearly bleeding episodes, and the number of infusions used to treat bleeding episodes.

In the study, children treated prophylactically with ALPROLIX had an overall median ABR of 1.97. The median ABR for spontaneous joint bleeds was zero. Approximately 33 percent of participants in the study experienced zero bleeding episodes. Overall, 91.7 percent of bleeding episodes were controlled by one or two infusions of ALPROLIX. The terminal half-life of ALPROLIX in the study was 66.5 hours for children under six and 70.3 hours for children six to less than 12 years of age. Additional analyses of the Kids B-LONG study are ongoing, and detailed results will be presented at a future scientific meeting.

No inhibitors to ALPROLIX were detected during the study. ALPROLIX was generally well tolerated and no cases of serious allergic reactions or vascular thrombotic events were reported in any participants, all of whom had been previously treated with other commercially available factor IX products. No serious adverse events were determined by any investigator to be related to the drug. One adverse event, decreased appetite, was considered related to ALPROLIX treatment and was reported in one participant. No participant discontinued the study due to an adverse event after receiving ALPROLIX. The pattern of treatment-emergent adverse events reported was consistent with the population studied and generally consistent with results seen in adolescents and adults in the pivotal Phase 3 B-LONG study.

"Sobi and Biogen Idec are committed to advancing treatment options for adults and children with hemophilia," said Birgitte Volck, M.D., Ph.D., senior vice president of development and chief medical officer at Sobi. "The completion of Kids B-LONG marks an important milestone in the ALPROLIX development program. We are excited to pursue the next stages of preparation for regulatory filing in Europe and to potentially advance treatment options for people with hemophilia B."

### About Kids B-LONG

Kids B-LONG was a global, open-label, multicenter Phase 3 study involving 30 boys with severe hemophilia B (factor IX activity equal to or less than 2 IU per dL, or 2 percent) with at least 50 prior exposure days to factor IX therapies. The study was conducted at 16 hemophilia treatment centers in six countries. Overall, 27 participants (90 percent) completed the study. The median time participants spent in the study was 49.4 weeks, and 24 participants received ALPROLIX infusions on at least 50 separate days (exposure days).

### About ALPROLIX

ALPROLIX® [Coagulation Factor IX (Recombinant), Fc Fusion Protein] is the only approved recombinant, clotting factor IX therapy with prolonged circulation in the body. In the United States, it is indicated for the control and prevention of bleeding episodes, perioperative (surgical) management and routine prophylaxis in adults and children with hemophilia B. ALPROLIX is not indicated for immune tolerance induction therapy, which is a treatment for people with inhibitors, and should not be used in individuals with a known history of allergic reactions to ALPROLIX or any of the other ingredients in ALPROLIX. ALPROLIX was developed by fusing factor IX to the Fc portion of immunoglobulin G subclass 1, or IgG<sub>1</sub> (a protein commonly found in the body). It is believed that this enables ALPROLIX to use a naturally occurring pathway to prolong the time the therapy remains in the body. While Fc fusion has been used for more than 15 years, Biogen Idec is the only company to apply it in hemophilia.

Common adverse reactions (incidence of greater than or equal to 1 percent) from the B-LONG study were headache and oral paresthesia (an abnormal sensation in the mouth). For complete prescribing information, go to [www.ALPROLIX.com](http://www.ALPROLIX.com).

ALPROLIX is also approved for the treatment of hemophilia B in Canada, Japan, and Australia.

## About Hemophilia B

Hemophilia B occurs in about one in 25,000 male births annually, and more rarely in females, affecting about 4,000 people in the United States. The World Federation of Hemophilia global survey conducted in 2012 estimates that approximately 28,000 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 bleeding episodes that cause pain, irreversible joint damage and life-threatening hemorrhages. Prophylactic infusions of factor IX temporarily replace clotting factors necessary to control bleeding and prevent new bleeding episodes.

## About the Biogen Idec and Sobi Collaboration

Biogen Idec and Sobi are collaborators in the development and commercialization of ALPROLIX for hemophilia B. Sobi has an opt-in right to take over exclusive final development and commercialization of ALPROLIX for the Sobi territories (Europe, North Africa, Russia and certain Middle Eastern markets). Biogen Idec leads development for ALPROLIX, has manufacturing rights, and has commercialization rights in North America and all other regions in the world excluding the Sobi territory.

## 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, hematologic conditions and autoimmune disorders. Founded in 1978, Biogen Idec is the world's oldest independent biotechnology company and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies. For product labeling, press releases and additional information about the Company, please visit <http://www.biogenidec.com>.

## 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 for partner companies across Europe, the Middle East, North Africa and Russia. Sobi is a pioneer in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2014, Sobi had total revenues of SEK 2.6 billion (USD 380 M) and about 600 employees. The share (STO: SOBI) is listed on NASDAQ OMX Stockholm. More information is available at [www.sobi.com](http://www.sobi.com).

## Biogen Idec Safe Harbor

This press release contains forward-looking statements, including statements about the potential and therapeutic impact of ALPROLIX. 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. 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, European regulatory authorities may require additional information or further studies, or may fail to approve, or refuse to approve, or may delay approval of our marketing authorization application for ALPROLIX, 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.



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