



## Biogen Idec and Sobi Announce Positive Top-Line Efficacy and Safety Results from Phase 3 Pediatric Study of Investigational Therapy ELOCTATE™ (rFVIII Fc) for Hemophilia A

April 10, 2014

*-Data Showed Twice-Weekly Prophylactic Dosing with ELOCTATE Maintained Low Bleeding Rates in Children Under Age 12-*

*-Study Meets Primary Objectives; Results Enable Regulatory Submission in EU-*

CAMBRIDGE, Mass. & STOCKHOLM--([BUSINESS WIRE](#))--[Biogen Idec](#) (NASDAQ: BIIB) and [Swedish Orphan Biovitrum AB](#) (publ) (Sobi) (STO: SOBI) today released positive top-line results of the Kids A-LONG Phase 3 clinical study that evaluated the safety and efficacy of ELOCTATE™, an investigational recombinant factor VIII Fc fusion protein product candidate, in children with severe hemophilia A. ELOCTATE was generally well-tolerated and no inhibitors (neutralizing antibodies that may interfere with the activity of the therapy) were detected. Efficacy analyses showed twice-weekly prophylactic dosing with ELOCTATE maintained low bleeding rates in children.

Kids A-LONG is the first study to evaluate a long-lasting, investigational hemophilia therapy in children under 12 years old. ELOCTATE was developed using a process called Fc fusion and is designed to prolong the circulation of infused clotting factor in the body. The successful completion of this study will support applications for pediatric indications globally and is an important step to obtaining marketing authorization in Europe. The European Medicines Agency requires the inclusion of pediatric study data in a marketing application for a new hemophilia therapy.

"Prophylactic treatment is recommended for children with severe hemophilia due to its proven health benefits. However, current prophylactic infusion schedules are challenging for many children with hemophilia A and their parents," said Glenn Pierce, M.D., Ph.D., senior vice president of Hematology, Cell and Gene Therapies at Biogen Idec. "The results of this study support the potential for ELOCTATE to address a significant need for children with hemophilia A by providing prolonged intervals between scheduled prophylactic infusions to protect against bleeding episodes."

Kids A-LONG investigated the safety, efficacy and pharmacokinetics (measurement of the presence of the drug in a person's body over time) of ELOCTATE in previously treated children under 12 years old with severe hemophilia A. The primary endpoint of the study was to evaluate the frequency of inhibitor (neutralizing antibody) development and none were detected. Secondary endpoints included the overall and spontaneous annualized bleeding rates (ABR), or projected number of yearly bleeding episodes, and the number of infusions used to prevent and treat bleeding episodes.

In the study, the relative increase in half-life in children with severe hemophilia A was consistent with the 1.5-fold increase in half-life seen in the A-LONG study of adults and adolescents. Children treated prophylactically with ELOCTATE had an overall median ABR of 2.0 and a median ABR for spontaneous bleeds of 0.0. Forty-six percent of participants in the study experienced zero bleeding episodes. Overall, ninety three percent of bleeding episodes were controlled by one to two infusions of ELOCTATE. Additional analyses of the Kids A-LONG study are ongoing, and the companies plan to present detailed results at a future scientific meeting.

"Sobi and Biogen Idec recognize the importance of advancing treatment options for adults and children with hemophilia," said Birgitte Volck, M.D., Ph.D., senior vice president development and chief medical officer of Sobi. "The successful completion of the Kids A-LONG study in children under 12 years old is an important step to bringing this potential new treatment option to adults and children with hemophilia A and a milestone that will enable regulatory submission in Europe."

### **About Kids A-LONG**

Kids A-LONG was a global, open-label, multi-center Phase 3 study involving 71 boys with severe hemophilia A (factor VIII activity less than 1 IU per dL, or 1 percent) with at least 50 prior exposure days to factor VIII therapies. The study was conducted at 23 hemophilia treatment centers in eight countries. Overall, 67 participants (94 percent) completed the study (33 under six years old and 34 six to 11 years old). The average time participants spent in the study was 25 weeks and 61 participants received ELOCTATE infusions on at least 50 separate days (exposure days) to assess inhibitor development.

All study participants were to be initially treated with twice-weekly prophylactic infusions of ELOCTATE [25 IU/kg day 1, 50 IU/kg day 4]. Study investigators could adjust the dose or interval based on individual response. Approximately ninety percent of study participants were on twice-weekly dosing at the end of the study.

ELOCTATE was generally well-tolerated. No inhibitors to ELOCTATE were detected and no cases of serious allergic reactions were reported in any participants, all of whom switched from commercially available factor VIII products. No serious adverse events were assessed to be related to drug by the investigator. Two non-serious events, rash and myalgia (muscle pain), were considered related to ELOCTATE treatment and were reported in one participant each. No participant discontinued the study due to an adverse event after receiving ELOCTATE. The pattern of treatment-emergent adverse events reported was typical of the population studied and generally consistent with results seen in adolescents and adults in the A-LONG study.

### **About ELOCTATE**

ELOCTATE is an investigational, recombinant clotting factor therapy developed for hemophilia A by fusing factor VIII 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 ELOCTATE to use a naturally occurring pathway to prolong the time 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 to the treatment of hemophilia.

Regulatory applications for ELOCTATE approval are currently under review in several countries including the United States, Australia, Canada and Japan.

## 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 recurrent and extended bleeding episodes that cause pain and irreversible joint damage. Some of these bleeding episodes can be life-threatening. According to the World Federation of Hemophilia, an estimated 142,000 people worldwide are identified living with hemophilia A.<sup>1</sup> Prophylactic infusions of factor VIII can temporarily replace the clotting factor necessary to control bleeding and prevent new bleeding episodes.

Inhibitor development is a response of the body's immune system that interferes with the activity of therapy. About 25 to 30 percent of people with severe hemophilia A develop inhibitors during their lifetime. Inhibitors typically develop after a median of 8-10 exposure days (EDs), though this number varies widely.<sup>2</sup>

## About the Biogen Idec and Sobi Collaboration

Biogen Idec and [Swedish Orphan Biovitrum \(Sobi\)](#) are partners in the development and commercialization of ELOCTATE for hemophilia A. Biogen Idec leads development, has manufacturing rights, and has commercialization rights in North America and all other regions in the world excluding the Sobi territory. Sobi has the right to opt in to assume final development and commercialization in Europe, 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. For product labeling, press releases and additional information about the company, please visit [www.biogenidec.com](http://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 Haemophilia 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 2013, Sobi had total revenues of SEK 2.2 billion (€253 M) and about 550 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 therapeutic effect of ELOCTATE, our recombinant factor VIII Fc fusion protein product 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.

<sup>1</sup> World Federation of Hemophilia. Annual Global Survey 2012. <http://www1.wfh.org/publications/files/pdf-1574.pdf>. Accessed January 28, 2014.

<sup>2</sup> Mariani G, Konkle BA, Kessler CM. Inhibitors in Hemophilia A and B. In: Hoffman R, ed. *Hematology : basic principles and practice*. 6th ed. Philadelphia, PA: Saunders/Elsevier; 2013:1961-1970.

## Multimedia Files:

[Download All Files](#)  
[Preview image](#)

Download:

[Download Square](#) (8.3 KB)  
[Download Square](#) (8.3 KB)  
[Download Web Ready](#) (35.96 KB)  
[Download Thumbnail](#) (2.86 KB)  
[Download High Resolution](#) (72.67 KB)  
[Download ViewImage](#) (7.66 KB)

## Contact:

BIOGEN IDEC:

Media Contact:

Lee-Ann Murphy, +1-781-464-3260

[public.affairs@biogenidec.com](mailto:public.affairs@biogenidec.com)

or

Investor Relations Contact:

Ben Strain, +1-781-464-2442

[IR@biogenidec.com](mailto:IR@biogenidec.com)

or

SOBI:

Media Contact:

Oskar Bosson, +46-70-410-71 80

[oskar.bosson@sobi.com](mailto:oskar.bosson@sobi.com)

or

Analyst/Investor Contact:

Jörgen Winroth, +1 347-224-0819

+46-8-697-2135

[jorgen.winroth@sobi.com](mailto:jorgen.winroth@sobi.com)