



## Biogen Idec and Swedish Orphan Biovitrum Present Data on Long-Lasting Recombinant Factor VIII Therapy at International Society on Thrombosis and Haemostasis Meeting

July 26, 2011

*--Results Show Potential to Significantly Reduce the Burden of Treatment for People with Hemophilia A--*

KYOTO, Japan--([BUSINESS WIRE](#))--[Biogen Idec](#) (NASDAQ: BIIB) and [Swedish Orphan Biovitrum](#) (STO: SOBI) today announced Phase 1/2a trial data showing that the companies' long-lasting fully-recombinant factor VIII Fc fusion protein (rFVIII<sub>Fc</sub>) was well tolerated and demonstrated an approximately 1.7-fold increase in half-life compared with Advate<sup>®</sup> (antihemophilic factor recombinant, plasma/albumin-free method, rFVIII), a commercially-available factor VIII product, in 16 previously-treated patients with severe hemophilia A. The findings, which were seen consistently across all patients and dose levels, are being presented today at the XXIIIrd Congress of the International Society on Thrombosis and Haemostasis in Kyoto, Japan.

"This trial is a step toward addressing the significant unmet need for a long-lasting recombinant factor VIII product," said Neil Josephson, M.D., Co-Principal Investigator of the Phase 3 rFVIII<sub>Fc</sub> A-LONG trial and associate professor of Medicine in the Division of Hematology at the University of Washington School of Medicine, Seattle, Wash. "Results from the Phase 1/2a study show that rFVIII<sub>Fc</sub> has an extended half-life, which may have the potential to provide extended protection from bleeding and reduce the number of infusions necessary for prophylactic treatment of severe hemophilia A."

Currently, prophylactic treatment of severe hemophilia A requires intravenous infusions three times a week or every other day. rFVIII<sub>Fc</sub> is a fully-recombinant and fully-active clotting factor designed to replace the protein that hemophilia A patients lack and to last longer in the body than commercially-available factor VIII products. Developed using Biogen Idec's proprietary Fc fusion technology, rFVIII<sub>Fc</sub> utilizes a natural pathway that recycles rFVIII<sub>Fc</sub> in the circulation to extend its half-life.

"Biogen Idec is driven to deliver innovative treatments that can make much-needed progress for people with hemophilia," said Glenn Pierce, M.D., Ph.D., Senior Vice President of Hemophilia at Biogen Idec. "These study results demonstrate the potential of our Fc fusion technology to develop long-lasting clotting factors that may significantly reduce the burden of treatment and improve quality of life for people with hemophilia."

"These results are promising and supported the advancement of rFVIII<sub>Fc</sub> into a Phase 3 trial last year," said Peter Edman, Ph.D., Chief Scientific Officer of Swedish Orphan Biovitrum. "We are excited about the potential of rFVIII<sub>Fc</sub> to make a positive impact on the health and quality of life of hemophilia A patients by providing extended protection from bleeding."

rFVIII<sub>Fc</sub> is currently being studied in a registrational, open-label, multicenter trial (A-LONG), which is evaluating its safety, pharmacokinetics and efficacy in the prevention and treatment of bleeding in previously-treated patients with severe hemophilia A.

Additionally, the European Medicines Agency's (EMA) Pediatric Committee recently adopted an opinion agreeing to the pediatric investigational plan for rFVIII<sub>Fc</sub>. In accordance with the opinion, Biogen Idec and Swedish Orphan Biovitrum plan to initiate a global pediatric trial in previously-treated patients under 12 years of age as soon as sufficient data are available from a study of older patients. Under draft guidelines published by the EMA for the development of factor VIII products, pediatric data from this trial will be required in the initial submission of a Marketing Authorization Application to the European regulatory agency.

### About the Phase 1/2a Study

The Phase 1/2a open-label, cross-over, multi-center, dose-escalation study evaluated the safety and pharmacokinetics of an intravenous injection of rFVIII<sub>Fc</sub> in 16 previously-treated patients with severe hemophilia A. The primary objective of the study was to assess the safety of rFVIII<sub>Fc</sub> at different doses; the secondary objective was to estimate the pharmacokinetic parameters of rFVIII<sub>Fc</sub> at doses ranging from 25 to 65 IU/kg.

rFVIII<sub>Fc</sub> was well tolerated in this single-dose study, with no drug-related serious adverse events. Adverse events were observed in 11 out of 16 patients, with one related to study drug – dysgeusia (abnormal taste in the mouth). There were no signs of injection site reactions, inhibitor development or anti-rFVIII<sub>Fc</sub> drug antibodies.

rFVIII<sub>Fc</sub> demonstrated an approximately 1.7-fold increase in half-life compared to Advate. Other PK parameters such as mean residence time and area under the curve (AUC) were similarly increased. Furthermore, peak plasma concentration and AUC also demonstrated an increase proportional to the dose administered relative to Advate. Advate and rFVIII<sub>Fc</sub> had comparable and dose-dependent peak plasma concentration, and comparable recovery.

### About Fc Fusion Technology and the Long-Lasting Recombinant Hemophilia Program

Developed using Biogen Idec's proprietary Fc fusion technology, rFVIII<sub>Fc</sub> utilizes a natural pathway that recycles rFVIII<sub>Fc</sub> in the circulation to extend its half-life and allow factor to remain in the body longer after an infusion.

Using the same, natural Fc fusion technology as rFVIII<sub>Fc</sub>, Biogen Idec and Swedish Orphan Biovitrum are also developing a fully-recombinant, long-lasting Factor IX Fc fusion protein (rFIX<sub>Fc</sub>) for the treatment of hemophilia B. rFIX<sub>Fc</sub> is currently being tested in a registrational, open-label, multicenter trial (B-LONG), which is designed to evaluate its safety, pharmacokinetics and efficacy in prevention and treatment of bleeding in hemophilia B patients. For more information on the rFVIII<sub>Fc</sub> and rFIX<sub>Fc</sub> trials, please visit [www.biogenidechemophilia.com](http://www.biogenidechemophilia.com) or [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### About Hemophilia A

Hemophilia A is a rare, inherited disorder in which the ability of a person's blood to clot is impaired. Hemophilia A occurs in about one in 5,000 male births annually and is caused by having substantially reduced or no factor VIII protein, which is needed for normal blood clotting. People with

hemophilia A therefore need injections of factor VIII to restore the coagulation process and prevent frequent bleeds that could otherwise lead to pain, irreversible joint damage and life-threatening hemorrhages. Prophylactic treatment with infusions three times per week or every other day to maintain a sufficient circulating level of coagulation factor is being increasingly used, and long-term studies demonstrate that such regimens increase the patient's life expectancy and greatly reduce, if not eliminate, progressive joint deterioration.

#### **About Biogen Idec**

Biogen Idec uses cutting-edge science to discover, develop, manufacture and market therapies for serious diseases with a focus on neurology, immunology and hemophilia. 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 \$4 billion in annual revenues. For product labeling, press releases and additional information about the company, please visit [www.biogenidec.com](http://www.biogenidec.com).

#### **About Swedish Orphan Biovitrum (Sobi)**

Sobi is a leading European specialty pharmaceutical company focused on providing and developing specialty pharmaceuticals for patients with rare diseases and significant medical needs. The portfolio comprises about 60 marketed products, as well as projects in late clinical phase. Key therapeutic areas are hematological diseases, autoimmune diseases, hereditary metabolic disorders and therapeutic oncology. In 2010 Sobi had revenues of SEK 1.9 billion and approximately 500 employees. The share (STO: SOBI) is listed on NASDAQ OMX Stockholm. For more information please visit [www.sobi.com](http://www.sobi.com).

#### **Safe Harbor**

This press release contains forward-looking statements, including statements about the development and potential effects of long-lasting hemophilia therapies. These statements may be identified by words such as "believe," "expect," "may," "plan," "will" and similar expressions, and are based on the companies' current beliefs and expectations. Drug development and commercialization involve a high degree of risk. Factors which could cause actual results to differ materially from the companies' current expectations include the risk that we may not fully enroll our planned clinical trials, unexpected concerns may arise from additional data or analysis, regulatory authorities may require additional information, further studies, or may fail to approve our drug candidates, or the companies may encounter other unexpected hurdles. For more detailed information on the risks and uncertainties associated with Biogen Idec's drug development and commercialization activities, please review the Risk Factors section of Biogen Idec'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 the companies assume no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

*Swedish Orphan Biovitrum may be required to disclose the information provided herein pursuant to the Swedish Securities Markets Act. The information was provided for public release on July 26, 2011 at 6:30 a.m. CET.*

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