



New Phase 3 Data Reinforce Long-Lasting Protection from Bleeding for Patients with Hemophilia A and B

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– Recombinant Fc fusion proteins show potential to transform care by providing long-lasting protection from bleeding with fewer injections than the current standard of care –

WARSAW, Poland--([BUSINESS WIRE](#))--[Biogen Idec](#) (NASDAQ: BIIB) and [Swedish Orphan Biovitrum](#) (Sobi) (STO: SOBI) released data that confirmed the ability of investigational recombinant factors VIII Fc fusion protein (rFVIII Fc) and IX Fc fusion protein (rFIX Fc) to provide long-lasting protection from bleeding with fewer injections than are required with the current standard of care for people with hemophilia. The data, from the largest phase 3 registrational studies conducted in hemophilia to date, were presented this week at the 6th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD).

The studies compared the pharmacokinetic activity of rFVIII Fc for hemophilia A and rFIX Fc for hemophilia B to currently available treatments. In the studies, the long-lasting candidates stayed active in the body longer, enabling study participants to prevent bleeding with less frequent injections than are required with the current standard of care. In the A-LONG study, patients with hemophilia A were able to use once to twice weekly prophylactic (preventative dosing) injections of rFVIII Fc while maintaining low bleeding rates. In the B-LONG study, rFIX Fc allowed patients with hemophilia B to use prophylactic injections every one to two weeks with low bleeding rates.

"Data from these phase 3 trials demonstrate a potential to transform the treatment of hemophilia by offering long-lasting protection from bleeding while meaningfully reducing treatment burden associated with this rare disease," said Glenn Pierce, M.D., Ph.D., senior vice president of Global Medical Affairs and chief medical officer of Biogen Idec's hemophilia therapeutic area. "Less frequent injections may help more people with hemophilia adhere to a preventative treatment schedule, which can help prevent the long-term health consequences associated with treating a bleed after it occurs."

The current standard of care for hemophilia A and B requires frequent injections, which are a burden for patients. Prophylactic treatment for hemophilia A typically requires injections three times per week or every other day, and injections 2-3 times per week for the treatment of hemophilia B, according to the National Hemophilia Foundation's Medical and Scientific Advisory Council guidelines. People with severe hemophilia who do not follow a prophylactic injection schedule remain vulnerable to bleeding that can cause irreversible joint damage and life-threatening hemorrhages.

Recombinant FVIII Fc and recombinant FIX Fc were developed using Fc fusion technology, which has safely been used in FDA-approved medicines for more than a decade. Biogen Idec and Swedish Orphan Biovitrum applied Fc fusion technology in hemophilia for the first time with the goal of making clotting factors last longer and reduce the burden of injections for patients and their families.

About the A-LONG (rFVIII Fc) Data at EAHAD

The A-LONG results confirm the long-lasting characteristics of rFVIII Fc; specifically, the data show that rFVIII Fc stays in the body for 50 percent longer than Advate® [antihemophilic factor (recombinant), plasma/albumin-free method], the most frequently used factor VIII therapy. In the trial, the terminal half-life for rFVIII Fc was 19 hours compared to 12 hours for Advate. Other measures of rFVIII Fc's activity in the body reinforce its long-lasting characteristics: the mean time for maintaining a clotting factor activity level associated with less bleeding (time to 1 percent) was approximately 5 days for rFVIII Fc compared to 3.5 days for Advate and the average rate at which rFVIII Fc was cleared from the body was 2.0 mL/hr/kg compared with 3.0 mL/hr/kg for Advate. In the study's individualized prophylaxis arm, patients received rFVIII Fc at a median dosing interval of 3.5 days and a median weekly dose of 78 IU per kg to prevent bleeding, which compares favorably to the recommended dose for the standard of care. Nearly one-third of patients were able to achieve every 5 day dosing in this arm. Overall, the A-LONG data indicate that rFVIII Fc has the potential to become the first product to offer hemophilia A patients long-lasting protection from bleeding with less frequent dosing than the current standard of care.

The A-LONG data were presented in the late-breaking oral abstract session and in poster 104, "Phase 3 clinical study of recombinant FC fusion factor FVIII (rFVIII Fc) demonstrated safety, efficacy, and improved pharmacokinetics (A-LONG)."

About the B-LONG (rFIX Fc) Data at EAHAD

The B-LONG results confirm the long-lasting characteristics of rFIX Fc; specifically, the data show that rFIX Fc stays in the body for more than twice as long as BeneFIX® [Coagulation Factor IX (Recombinant)], the only recombinant factor IX therapy currently approved for prophylactic use. The terminal half-life for rFIX Fc was 82 hours compared to 34 hours for BeneFIX. Other measures of rFIX Fc's activity in the body reinforce its long-lasting characteristics: the mean time for maintaining a normal clotting factor activity level (time to 1 percent) was 11 days for rFIX Fc compared to 5 days for BeneFIX and the average rate at which rFIX Fc was cleared from the body was 3.2 mL/hr/kg compared with 6.3 mL/hr/kg for BeneFIX. All patients in the individualized interval prophylaxis arm of the study were able to go at least one week between rFIX Fc injections and 50 percent were able to go 14 days or longer before needing another dose to prevent bleeding. The median weekly dose was 45 IU per kg, comparable to the recommended dose for the current standard of care. Overall, the B-LONG data support the potential for rFIX Fc to become the first product to offer hemophilia B patients long-lasting protection from bleeding with a more convenient injection schedule than the current standard of care.

The B-LONG data were presented in poster 115, "Safety, efficacy, and improved pharmacokinetics (PK) demonstrated in a phase 3 clinical trial of extended half-life recombinant FC fusion factor IX (B-LONG)."

Importantly, the difference in the duration of activity of rFVIII Fc and rFIX Fc was expected and consistent with the differences between the natural clotting factors that these products augment. Fc Fusion technology extends FVIII and FIX differently based on the biological differences in hemophilia A and B. The length of time that FVIII stays active is dictated by its own duration as well as that of the blood protein that it binds to, known as von Willebrand factor. The activity of FIX is not restricted in this way.

"These new data support the application of Fc fusion technology in hemophilia, using a naturally occurring pathway to delay the breakdown of factor in the body and cycle it back into the bloodstream," said Birgitte Volck, M.D., Ph.D., senior vice president & chief medical officer of Sobi. "While Fc fusion has been used in medicines for more than a decade, rFVIII Fc and rFIX Fc are the first investigational therapies to use the technology to successfully

extend the half-lives of clotting factors, which could offer protection from bleeding while reducing the burden of injections for people with hemophilia.”

About the A-LONG Study and the rFVIII Fc Program

A-LONG was a global, open-label, multi-center phase 3 study that evaluated the efficacy, safety and pharmacokinetics of intravenously-injected rFVIII Fc in 165 male patients aged 12 years and older. The study results, first announced in October 2012, showed that rFVIII Fc was effective in the control and prevention of bleeding, routine prophylaxis and perioperative management, with low single-digit median annualized bleeding rates using individualized and weekly prophylactic regimens. Overall, 98 percent of bleeding episodes were controlled by one or two injections of rFVIII Fc. Recombinant FVIII Fc was generally well-tolerated and no inhibitors to rFVIII Fc were detected. The most common adverse events (incidence of ≥ 5 percent) occurring outside of the perioperative management period were nasopharyngitis, arthralgia, headache and upper respiratory tract infection. No serious adverse events were assessed to be related to the therapy by the investigators.

Ongoing clinical studies of rFVIII Fc include Kids A-LONG, for previously-treated children with hemophilia A under age 12, and ASPIRE, for patients who completed the A-LONG study or who complete the Kids A-LONG study.

About the B-LONG Study and the rFIX Fc Program

B-LONG was a global, open-label, multi-center phase 3 study that evaluated the efficacy, safety and pharmacokinetics of intravenously-injected rFIX Fc in 123 male patients aged 12 years and older. The study results, first announced in September 2012, showed that rFIX Fc was effective in the control and prevention of bleeding, routine prophylaxis, and perioperative management, with low single-digit median annualized bleeding rates using individualized prophylactic regimens at a median dosing interval of 14 days. More than 90 percent of bleeding episodes were controlled by a single injection of rFIX Fc. Recombinant FIX Fc was generally well-tolerated and no inhibitors to rFIX Fc were detected. The most common adverse events (incidence of ≥ 5 percent) occurring outside of the perioperative management arm (i.e., Arms 1, 2 and 3, but not Arm 4) were nasopharyngitis, influenza, arthralgia (joint pain), upper respiratory infection, hypertension and headache. One serious adverse event, obstructive uropathy in the setting of hematuria, was assessed to be possibly related to therapy by the investigator. The patient continued rFIX Fc treatment and the event resolved with medical management.

Ongoing clinical studies of rFIX Fc include Kids B-LONG, for previously treated children with hemophilia B under age 12, and B-YOND, for patients who completed the B-LONG study or who complete the Kids B-LONG study.

About the Fc Fusion Technology Platform

Recombinant FVIII Fc and recombinant FIX Fc are clotting factors developed using Biogen Idec's novel and proprietary monomeric Fc fusion technology, which makes use of a naturally occurring pathway that delays the breakdown of factor in the body and cycles it back into the bloodstream, enabling it to remain in the body longer following an injection. Fc fusion technology is used in seven FDA-approved products for the long-term treatment of chronic diseases including rheumatoid arthritis, psoriasis and platelet disorders.

About Hemophilia

Hemophilia is a rare, inherited disorder in which the ability of a person's blood to clot is impaired. Hemophilia A is caused by reduced or no Factor VIII protein, whereas hemophilia B is caused by reduced or no Factor IX protein. Both proteins are needed for normal blood clotting. Hemophilia A and hemophilia B occur in about one in 5,000 and one in 25,000 male births, respectively. People with hemophilia need injections of clotting factors to restore the coagulation process and prevent frequent bleeds that could otherwise lead to pain, irreversible joint damage and life-threatening hemorrhages. The Medical and Scientific Advisory Council of the National Hemophilia Foundation recommends prophylaxis as the optimal therapy for people with severe hemophilia A and severe hemophilia B. Currently, prophylaxis for hemophilia A typically requires injections three times per week or every other day to maintain a sufficient circulating level of clotting factor, while prophylaxis in hemophilia B typically requires injections two to three times per week.

About the Biogen Idec and Sobi Collaboration

Biogen Idec and Sobi are partners in the development and commercialization of rFVIII Fc and rFIX Fc. 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, 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 healthcare company dedicated to bringing innovative therapies and services to improve the lives of rare disease patients. The product portfolio is primarily focused on inflammation and genetic diseases, with three late stage biological development projects within hemophilia and neonatology. Sobi also markets more than 40 products for companies in the specialty and rare disease space. In 2011, Sobi had revenues of SEK 1.9 billion and around 500 employees. The share (STO: SOBI) is listed on NASDAQ OMX Stockholm. More information is available at www.sobi.com.

Safe Harbor

This press release contains forward-looking statements, including statements about the commercialization and impact of long-lasting hemophilia therapies. 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 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 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 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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