Results from B-YOND study reinforce long-term clinical profile of Alprolix for the treatment of haemophilia B

Swedish Orphan Biovitrum AB (publ) (STO: SOBI) and its partner Biogen released interim results from the B-YOND study presented at the 67th Annual Meeting for the National Hemophilia Foundation in Dallas, Texas. The results support the long-term safety and efficacy of Alprolix® (rFIXFc) in people with severe haemophilia B treated for up to two years. Participants in this phase 3, open-label long-term study maintained low bleeding rates with one to two week prophylaxis regimens. No inhibitors have been reported to-date.

“B-YOND represents the most extensive long-term clinical data set for a long-acting replacement FIX product. These interim results support the efficacy and safety profile of Alprolix in paediatric patients as well as in adults and adolescents”, says Birgitte Volck, Chief Medical Officer at Sobi.

B-YOND is an ongoing extension study for people with severe haemophilia B who completed the phase 3 pivotal B-LONG or Kids B-LONG study. The study’s primary endpoint is inhibitor development. From the beginning of B-LONG or Kids-B-LONG until the B-YOND interim data analysis, the cumulative median time on Alprolix was 171.6 weeks for adults and adolescents (n=93), and 95.3 weeks for children under age 12 (n=23).

Alprolix (rFIXFc) is an investigational recombinant clotting factor IX therapy designed to have prolonged circulation in the body. According to the interim analysis, adults and adolescents treated prophylactically maintained protection against bleeding episodes with injections every one to two weeks. These participants had overall median annualised bleeding rates (ABR) of 2.28 for weekly prophylaxis (20-100 IU/kg of Alprolix every seven days), 2.25 for individualised prophylaxis (100 IU/kg of Alprolix every 8 to 16 days, or every other week) and 2.42 for modified prophylaxis (personalised dosing if optimal prophylaxis could not otherwise be achieved). In contrast, people receiving on-demand therapy, or treatment when a bleeding episode occurred, had a median ABR of 11.27.

The median overall ABR was zero for children under age six who received weekly prophylaxis (n=9). For children six to 12 years old, median overall ABRs were 2.65 (n=10), 2.37 (n=5) and 3.13 (n=1) in weekly, individualised and modified prophylaxis regimens, respectively. In each age group, the median average weekly dose for participants previously on prophylaxis was similar for individuals in the weekly and individualised treatment arms.
“Study participants receiving prophylactic treatment continue to experience low bleeding rates during this long-term study. The B-YOND study plays an important role in helping us understand this therapy’s long-term clinical profile and therapeutic value,” says Professor John Pasi, Professor of Haemostasis and Thrombosis at Barts and the London, United Kingdom.

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About Haemophilia B
Approximately 28,500 people are diagnosed with haemophilia B worldwide, according to World Hemophilia federation. Haemophilia B is caused by substantially reduced or no factor IX activity, which is needed for normal blood clotting. People with haemophilia B experience bleeding episodes that cause pain, irreversible joint damage, and life-threatening haemorrhages. Prophylactic injections of factor IX temporarily replace clotting factors necessary to control bleeding and prevent new bleeding episodes.

About Alprolix (rFIXFc)
Alprolix (rFIXFc) is a long-acting recombinant factor IX Fc fusion protein product candidate for people with haemophilia B. Alprolix [Coagulation Factor IX (Recombinant), Fc Fusion Protein], is the first recombinant, clotting factor therapy with prolonged circulation in the body for adults and children with haemophilia B, approved in the United States, Canada, Australia and Japan. Alprolix was submitted to the European Medicines Agency (EMA) for regulatory approval in Europe in June 2015. Alprolix was developed by fusing factor IX to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body). This enables Alprolix to use a naturally occurring pathway to prolong the time the therapy remains in the body.

About B-YOND
B-YOND is an ongoing long-term study for people with severe haemophilia B who completed the phase 3 pivotal B-LONG or Kids B-LONG studies. The study’s primary endpoint is inhibitor development. B-YOND enrolled 116 males, including 93 participants (81 per cent) who completed B-LONG, and 23 children (100 per cent) of those who completed Kids B-LONG. Secondary endpoints of the B-YOND study include ABRs (including spontaneous joint bleeding rates) per participant and treatment exposure days per participant. Additional outcomes are incidence of adverse events and serious adverse events, and evaluation of treatment of a bleeding episode (number of injections, dose per injection).

About Sobi and Biogen collaboration
Sobi and Biogen are collaboration partners in the development and commercialisation of Alprolix for haemophilia B. Sobi has recently exercised its opt-in right to assume exclusive final development and commercialisation rights of Alprolix in Europe, Russia, North Africa and certain countries in the Middle East. Biogen has led development for Alprolix, has manufacturing rights, and has commercialisation rights in North America and all other regions in the world excluding Sobi territory.

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 Haemophilia, Inflammation and Genetic diseases. We also market 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.
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