Biogen and Sobi Receive Positive Opinion from CHMP for ELOCTA™ (rFVIIIFc) for the Treatment of Hemophilia A

September 25, 2015

CAMBRIDGE, Mass. & STOCKHOLM--(BUSINESS WIRE)--Biogen (NASDAQ:BIIB) and Swedish Orphan Biovitrum AB (publ) (Sobi) (STO:SOBI) received a positive recommendation from the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) for the marketing authorization of ELOCTA ™ (rFVIIIFc). ELOCTA is a recombinant factor VIII Fc fusion protein product for the treatment of hemophilia A that, if approved, would be the first hemophilia A treatment with prolonged circulation available in the European Union (EU).

The positive opinion was based on results from the pivotal, Phase 3 A-LONG clinical study, which examined the efficacy, safety and pharmacokinetics of rFVIIIFc in previously treated males 12 years of age and older with severe hemophilia A, and from the Phase 3 Kids A-LONG clinical study, which evaluated the efficacy and safety of rFVIIIFc in previously treated male children with hemophilia A under 12 years of age. The Committee's positive opinion is now referred to the European Commission (EC), which grants marketing authorization for medicines in the EU.

"The CHMP's recommendation to approve ELOCTA is an important milestone in potentially bringing this innovative therapeutic option to people with hemophilia A across Europe," said Aoife Brennan, M.D, vice president of Hematology, Clinical Development at Biogen. "The potential of ELOCTA to provide protection against bleeding episodes with fewer prophylactic infusions will, if approved, represent the first treatment advance in nearly 20 years for Europe's hemophilia community."

ELOCTA is the European trade name for rFVIIIFc, which is also known as ELOCTATE® [Antihemophilic Factor (Recombinant), Fc Fusion Protein] in the U.S., Canada, Australia, New Zealand and Japan, where it is approved for the treatment of hemophilia A. Commonly reported adverse drug reactions (>/= 1% of subjects) in the clinical studies were arthralgia, malaise, myalgia, headache and rash. Development of Factor VIII neutralizing antibodies (inhibitors) may occur following administration of ELOCTA.

Biogen and Sobi are collaboration partners in the development and commercialization of ELOCTA/ELOCTATE for hemophilia A. Last year, Sobi exercised its opt-in right to assume final development and commercialization of ELOCTA in the Sobi territories (essentially, Europe, North Africa, Russia and certain countries in the Middle East). Biogen leads development for ELOCTA/ELOCTATE, has manufacturing rights, and has commercialization rights in North America and all other regions in the world excluding the Sobi territories.

"We are committed to bringing meaningful treatment advances to the hemophilia community in Europe and worldwide," said Birgitte Volck, M.D., Ph.D., senior vice president of Development and chief medical officer of Sobi. "We welcome the news of this positive CHMP opinion and look forward to the EC's forthcoming ELOCTA decision."

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 bleeding episodes that may cause pain, irreversible joint damage and life-threatening hemorrhages. According to the World Federation of Hemophilia, an estimated 140,000 people worldwide are identified as living with hemophilia A.[1]

About the A-LONG Clinical Study

The Phase 3 A-LONG clinical study was an open-label, multi-center study involving 165 previously treated males 12 years of age and older with severe hemophilia A. The study evaluated individualized and weekly prophylaxis to reduce or prevent bleeding episodes, and on-demand dosing to treat bleeding episodes. In the individualized arm, each study participant started on a twice-weekly dosing regimen. Participants' pharmacokinetic parameters were used to guide adjustments to dosing interval (every three to five days), and dose (25 to 65 IU/kg) to target a minimum factor VIII level of 1 to 3 IU/dL or higher as needed to prevent and control breakthrough bleeding episodes. In the study, the dose in the weekly prophylaxis arm was 65 IU/ kg/week.

About the Kids A-LONG Clinical Study

The Kids A-LONG study is the first clinical study to evaluate an investigational hemophilia therapy with a prolonged half-life in children younger than 12 years of age. The study was a global, open-label, multi-center Phase 3 study involving 71 boys with severe hemophilia A with at least 50 prior exposure days to factor VIII therapies.

Participants in both the A-LONG and Kids A-LONG clinical trials were able to enroll in ASPIRE, a Phase 3, open-label extension study evaluating the long-term safety and efficacy of ELOCTATE. For more information about these studies, please visit www.biogen.com.

About Biogen

Through cutting-edge science and medicine, Biogen discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hematologic conditions and autoimmune disorders. Founded in 1978, Biogen is one of the world's oldest independent biotechnology companies 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 www.biogen.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.

Biogen Safe Harbor

This press release contains forward-looking statements, including statements about the potential therapeutic impact and potential approvability of ELOCTA/ELOCTATE and the possible timing thereof. 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 refuse 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.

[1] **World Federation of Hemophilia. Annual Global Survey 2013. http://www1.wfh.org/publications/files/pdf-1591.pdf. Accessed September 2015.**

Multimedia Files:

Download All Files
Preview image

Download:
Download Thumbnail (7.96 KB)
Download Preview (10.75 KB)
Download Small (43.38 KB)
Download Full Size (53.87 KB)
Download Square (10.42 KB)
Preview image
http://www.sobi.com
Download:
Download Thumbnail (6.06 KB)
Download Preview (8.46 KB)
Download Small (36.76 KB)
Download Full Size (73.48 KB)
Download Square (8.92 KB)

Contact:

BIOGEN:
Media:
Lee-Ann Murphy, +1-781-464-3260
public.affairs@biogen.com
or
Investor Relations:
Ben Strain, +1-781-464-2442
IR@biogen.com
or
SOBI:
Media:
Oskar Bosson, +46-70-410-71 80
oskar.bosson@sobi.com
or
Analysts/Investors:
Jörgen Winroth, +1-347-224-0819
+46-8-697-2135
jorgen.winroth@sobi.com