Health Canada Approves Biogen Idec's Long-Acting ALPROLIX™ Therapy for Hemophilia B

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The Approval of ALPROLIX is First Significant Advance in Hemophilia B Treatment in More Than 17 Years

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Today Biogen Idec (NASDAQ: BIIB) announced that Health Canada has approved ALPROLIX ™ [Coagulation Factor IX (Recombinant), Fc Fusion Protein], for the control and prevention of bleeding episodes and routine prophylaxis in adults, and children aged 12 and older, with hemophilia B. ALPROLIX is the first approved long-acting hemophilia B therapy and is indicated to prevent or reduce the frequency of bleeding episodes with prophylactic (protective) infusions starting at once weekly or once every 10-14 days.

This is the first regulatory approval worldwide for ALPROLIX, which is currently under review by regulatory authorities in several other countries, including the United States, Australia and Japan.

"Health Canada's approval of ALPROLIX marks the first significant treatment advance in hemophilia B in 17 years, and reinforces our commitment to developing innovative therapies that help address the critical needs of the hemophilia community," said George A. Scangos, Ph.D., chief executive officer of Biogen Idec. "We believe that the safety, efficacy and prophylactic dosing schedule demonstrated with ALPROLIX will provide people with hemophilia B a meaningful new way to manage their condition."

The World Federation of Hemophilia recommends a prophylactic (prevention of bleeding) regimen as the goal of treatment for people with severe hemophilia.[1,2,3] Guidelines established by the Medical and Scientific Advisory Council of the National Hemophilia Foundation recommend prophylactic infusions two or more times a week with traditional hemophilia treatments.[4] Frequent prophylactic infusions can be a burden to people with hemophilia and may reduce adoption to this type of treatment regimen.[5]

The Health Canada approval of ALPROLIX is based on results from the global, Phase 3 B-LONG study, the largest registrational study in hemophilia B ever completed. It demonstrated that ALPROLIX safely and effectively prevented, or reduced, bleeding episodes with prophylactic infusions given once weekly or once every 10-14 days in adults and adolescents with severe hemophilia B. In addition, more than 90 percent of all bleeding episodes were controlled by a single ALPROLIX infusion.

"Health Canada's approval of ALPROLIX provides people with hemophilia B an important new option in maintaining a prophylactic regimen," said Manuel Carcao, M.D., Pediatric Hematologist and co-director of the Comprehensive Care Hemophilia Program at the Hospital for Sick Children in Toronto. "ALPROLIX, the first approved long-acting factor concentrate, has been shown to help individuals with hemophilia B achieve effective bleed prevention with prophylactic dosing once a week or once every 10 to 14 days."

Hemophilia B is a rare, chronic, inherited disorder in which the ability of a person's blood to clot is impaired, which can lead to recurrent and extended bleeding episodes. It is due to a substantial reduction of, or no factor IX activity, which is needed for normal blood clotting. People with hemophilia B experience bleeding episodes that can cause pain, irreversible joint damage and hemorrhage. Hemophilia B affects approximately one in 25,000 male births, or about 700 people in Canada.[6] The World Federation of Hemophilia global survey conducted in 2012 estimates that about 28,000 people are currently diagnosed with hemophilia B worldwide.

"The Canadian Hemophilia Society (CHS) is pleased that Health Canada has approved ALPROLIX, a second recombinant factor IX product to treat hemophilia B," said Craig Upshaw, CHS President. "Moreover, it is the first in a promising new class of factor products with extended half-life. We hope it will be available to Canadian patients in the very near future."

Biogen Idec is committed to helping people with hemophilia B and is working with Canadian Blood Services, the Service de Biovigilance du Ministère de la Santé et des Services sociaux and other provincial and national authorities to make ALPROLIX commercially available to all Canadians with hemophilia B.

About the B-LONG Study

B-LONG was a global, open-label, multi-center phase 3 study that evaluated the efficacy, safety and pharmacokinetics (measurement of the presence of the therapy in a patient's body over time), of ALPROLIX in 123 males aged 12 years and older with hemophilia B. These findings were published in the December 12, 2013 issue of *The New England Journal of Medicine*. The study involved 50 hemophilia treatment centers in 17 countries, on six continents.

The overall median annualized bleeding rates (ABR), or projected rate of bleeding episodes per year, reported in the study were 2.95 for the weekly prophylaxis arm and 1.38 for the individualized-interval prophylactic regimens arm, in which the dosing interval started at every 10 days, and 17.69 in the on-demand treatment arm. The overall median dosing interval with individualized-interval prophylaxis was 12.5 days. During the last six months of the study the median dosing interval was 13.8 days.

The most common adverse events (incidence of ≥5 percent in a pooled analysis of groups 1, 2, and 3) were nasopharyngitis (common cold), influenza (flu), arthralgia (joint pain), upper respiratory tract infection, hypertension (high blood pressure) and headache.

About ALPROLIX

ALPROLIX [Coagulation Factor IX (Recombinant), Fc Fusion Protein] is the first long-acting fully recombinant clotting factor therapy. It is indicated for the control and prevention of bleeding episodes and routine prophylaxis in adults, and children 12 years and older, with hemophilia B. ALPROLIX is developed by fusing factor IX to the Fc portion of Immunoglobulin G Subclass 1, or IgG^1 (protein commonly found in the body). It is believed that this enables ALPROLIX 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 in hemophilia.

The most common adverse drug reactions observed in the clinical trial (incidence ≥ 1%) were headaches and oral paresthesia (an abnormal sensation in the mouth).

About the Biogen Idec and Sobi Collaboration

Biogen Idec and Swedish Orphan Biovitrum (Sobi) are partners in the development and commercialization of ALPROLIX for hemophilia B. 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.

Biogen Idec Safe Harbor

This press release contains forward-looking statements, including statements about the potential and therapeutic impact of ALPROLIX. These forward-looking statements may be accompanied by such words as "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "potential," "project," "target," "will" and other words and terms of similar meaning. You should not place undue reliance on these statements. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including uncertainty of success in our commercialization of ALPROLIX, which may be impacted by, among other things, slower than anticipated acceptance of ALPROLIX by patients and the medical community, intense competition in the hemophilia market, the effectiveness of our sales force and marketing efforts, problems with the manufacturing process for ALPROLIX, the occurrence of adverse safety events, difficulties in obtaining, or changes in the availability of reimbursement for our products, our failure to obtain regulatory approvals in jurisdictions outside of Canada, including in the event other companies receive marketing approval of their treatments before approval of our treatments, our failure to protect our intellectual property and other proprietary rights, product liability claims and the other risks and uncertainties that are described in the Risk Factors section of our most recent annual or quarterly report and in other reports we have filed with the U.S. Securities and Exchange Commission (SEC). 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. 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] Nilsson IM, Berntorp E, Löfqvist T, Pettersson H. Twenty-five years' experience of prophylactic treatment in severe haemophilia A and B. J Intern Med 1992;232:25-32.

[2] Manco-Johnson MJ, Abshire TC, Shapiro AD, et al. Prophylaxis versus episodic treatment to prevent joint disease in boys with severe hemophilia. N Engl J Med 2007;357:535-44.

[3] Panicker J, Warrier I, Thomas R, Lusher JM. The overall effectiveness of prophylaxis in severe haemophilia. Haemophilia : the official journal of the World Federation of Hemophilia 2003;9:272-8.

[4] http://www.hemophilia.org/NHFWeb/MainPgs/MainNHF.aspx?menuid=57&contentid=1007

[5] Hacker MR, Geraghty S, Manco-Johnson M. Barriers to compliance with prophylaxis therapy in haemophilia. Haemophilia : the official journal of the World Federation of Hemophilia 2001;7:392-6.

[6] Canadian Hemophilia Registry. Hemophilia B. http://www.fhs.mcmaster.ca/chr/pdf/13/CHRIX130517b.pdf / Accessed February 18, 2014.

Contact:

Biogen Idec
Media Contact:
Lee-Ann Murphy, +1 781-464-3260
public.affairs@biogenidec.com
or
Canadian Media Contact:
Rick Maddalena, +1 416-486-7225
rick.maddalena@gcicanada.com
or
Investor Relations Contact:
Ben Strain, +1 781-464-2442
IR@biogenidec.com