Results from B-YOND Extension Study Reinforce Long-Term Clinical Profile of ALPROLIX for the Treatment of Hemophilia B

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Interim Data Presented at NHF Annual Meeting Show ALPROLIX Maintained Low Bleeding Rates with Prophylactic Infusions One to Two Weeks Apart

DALLAS--(BUSINESS WIRE)--New clinical data support the long-term safety and efficacy of ALPROLIX® [Coagulation Factor IX (Recombinant), Fc Fusion Protein] in people with severe hemophilia B treated for up to two years, Biogen (NASDAQ:BIIB) announced today. Participants in the Phase 3, open-label extension study, B-YOND, maintained low bleeding rates with one to two week prophylaxis regimens, according to data from an interim analysis. Investigators presented these interim results for the first time at the 67th Annual Meeting for the National Hemophilia Foundation (NHF) in Dallas.

B-YOND is a multi-year study for people with severe hemophilia B who completed the Phase 3 pivotal B-LONG or Kids B-LONG studies. In this interim analysis, the median time on ALPROLIX during B-YOND was 27.6 months for adults and adolescents (n=93), and 47.7 weeks for children under age 12 (n=23). The study's primary endpoint is inhibitor development, and no inhibitors have been reported to-date. There were three prophylactic dosing options for adult, adolescent, and pediatric participants in the B-YOND trial – weekly, individualized, and modified prophylaxis. An episodic treatment arm was also available for adult and adolescent patients.

"We believe B-YOND will play an important role in helping us understand this therapy's clinical profile over the long term," said Amy Shapiro, M.D., co-founder and medical director of the Indiana Hemophilia and Thrombosis Center. "Study participants receiving prophylactic treatment with intervals of one to two weeks between infusions continue to experience low bleeding rates during this ongoing extension study."

ALPROLIX is a 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 infusions every one to two weeks. These participants had overall median annualized bleeding rates (ABR) of 2.28 for weekly prophylaxis (20-100 IU/kg of ALPROLIX every seven days), 2.25 for individualized prophylaxis (100 IU/Kg of ALPROLIX every 8 to 16 days, or twice-monthly) and 2.42 for modified prophylaxis (personalized 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 between six to 12 years old, median overall ABRs were 2.65 (n=10), 2.37 (n=5) and 3.13 (n=1) in weekly, individualized 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 individualized treatment arms.

Safety results were typical of the hemophilia B populations studied. The most common adverse events (incidence of greater than or equal to five percent) included headache, common cold and vomiting for adults and adolescents. For children under age 12, falls, common cold and seasonal allergy were the most common adverse events.

Growing Body of Evidence Further Validates the Prolonged Half Life of ALPROLIX

In B-YOND, participants can change between treatment groups at enrollment, and at any time during the study. Most adult and adolescent participants remained in the same treatment group during B-YOND that they had participated in during B-LONG. The majority of children under age 12 stayed on once-weekly treatment (78 percent). Adults and adolescents were able to achieve a median dosing interval of 6.9 days in the modified prophylaxis arm, and 13.7 days in the individualized prophylaxis arm, while maintaining low ABRs.

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, and 95.3 weeks for children under age 12.

"The safety and efficacy of ALPROLIX with extended-interval prophylaxis dosing has been established across a robust clinical development program, where we consistently have observed low overall bleed rates, as well as low rates of spontaneous and joint bleeds," said Kate Dawson, vice president, U.S. Medical Affairs at Biogen. "These results of the B-YOND study provide additional insights supporting the efficacy and safety profile of ALPROLIX in adults and children with hemophilia B."

B-YOND enrolled 116 males, including 93 participants (81 percent) who completed B-LONG, and 23 (100 percent) 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 infusions, dose per infusion).

About ALPROLIX

ALPROLIX® [Coagulation Factor IX (Recombinant), Fc Fusion Protein], the first recombinant clotting factor therapy with prolonged circulation in the body, is approved in the United States, Canada, Australia and Japan. It is indicated in the United States for the control and prevention of bleeding episodes, perioperative (surgical) management and routine prophylaxis in adults and children with hemophilia B. ALPROLIX is not indicated for immune tolerance induction therapy, which is a treatment for people with inhibitors. The treatment should not be used in individuals with a known history of serious allergic reactions to ALPROLIX or its ingredients.

Introduced in the U.S. in May 2014 and Japan in September 2014, ALPROLIX provides protection from bleeding episodes with the potential to extend the interval between prophylactic infusions. The therapy was developed using a process called Fc fusion, which is designed to prolong a therapy's circulation in the body using a naturally occurring pathway. While Fc fusion has been used for more than 15 years, Biogen is the only company to apply

it to the treatment of hemophilia.

Development of neutralizing antibodies (inhibitors) to ALPROLIX may occur following administration of treatment. Common adverse reactions (incidence of greater than or equal to 1 percent) from the registrational B-LONG study were headache and oral paresthesia (an abnormal sensation in the mouth). For additional important safety information, and the United States full prescribing information, please visit www.ALPROLIX.com.

About Hemophilia B

Hemophilia B 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 IX. People with hemophilia B experience recurrent and extended bleeding episodes that cause pain and irreversible joint damage. Some of these bleeding episodes can be life-threatening. Hemophilia B occurs in about one in 25,000 male births annually, and more rarely in females, affecting about 4,000 people in the United States. The World Federation of Hemophilia global survey conducted in 2012 estimates that approximately 28,000 people are currently diagnosed with hemophilia B worldwide. Prophylactic infusions of factor IX temporarily replace clotting factors necessary to control bleeding and prevent new bleeding episodes. The Medical and Scientific Advisory Council of the National Hemophilia Foundation recommends prophylaxis as the optimal therapy for people with severe hemophilia B.

Inhibitor development is a response of the body's immune system that interferes with the activity of therapy. Approximately 2-3 percent of people with severe hemophilia B develop inhibitors during their lifetime. Inhibitors in people with hemophilia B are less common than in hemophilia A, however, the development of inhibitors can be associated with a severe allergic reaction that may be life-threatening.[1]

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.

Biogen Safe Harbor

This press release contains forward-looking statements, including statements about the safety and efficacy of ALPROLIX in people with hemophilia B treated with prophylaxis regimens of ALPROLIX. 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 or new 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 expanded indications or uses of our drug products, 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] Hemophilia Federation of America. Inhibitors. Available at: http://www.hemophiliafed.org/bleeding-disorders/inhibitors. Accessed August 2015.

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