Pfizer Announces Positive Top-Line Results From Phase 3 Study Of NONACOG ALFA (BeneFIX®) Once-Weekly Prophylaxis For Hemophilia B

Wednesday, July 16, 2014 - 03:00am

Once-Weekly Prophylaxis Treatment with BeneFIX® Significantly Reduced Annualized Bleeding Rate Compared to On-Demand Treatment

Pfizer Inc. (NYSE:PFE) today announced the positive results of a Phase 3 study comparing a prophylaxis regimen of BeneFIX® Coagulation Factor IX (Recombinant) 100 IU/kg once-weekly to on-demand treatment in people with moderately severe to severe hemophilia B. The top-line results of the study showed that the primary study endpoint was met and hemophilia B patients taking once-weekly BeneFIX (100 IU/Kg) showed a statistically significant reduction in the annualized bleeding rate (ABR) ($P < 0.0001$) relative to on-demand treatment with BeneFIX.

In the study, the median ABR value, a commonly used measure of efficacy for prophylaxis regimens in hemophilia, was 2.0 for the prophylaxis regimen, compared to 33.6 for the on-demand regimen, representing a 94% decrease in bleeding rates. The mean ABR value was 3.6 for the prophylaxis period, compared to 32.9 for the on-demand treatment, which represents a reduction of 89% ($P < 0.0001$).

“These results are important because they add to the growing body of clinical evidence showing that prophylaxis treatment has the potential to reduce the number of bleeds in a year, the most critical factor in hemophilia management,” said Steven J. Romano, M.D.,

senior vice president and Medicines Development Group Head, Global Innovative Pharmaceuticals, Pfizer Inc. “Pfizer remains committed to the research and development of new and innovative products for the hemophilia community.”

Study results also showed that prophylaxis treatment significantly reduced both spontaneous and traumatic ABR compared to on-demand treatment with BeneFIX. The median spontaneous ABR value was 1.0 for the prophylaxis regimen, compared to 22.4 for the on-demand regimen, and the median traumatic ABR value was 1.0 for the prophylaxis period, compared to 4.1 for the on-demand treatment. In addition to meeting the primary endpoint, the secondary study endpoints showed that none of the 1,254 prophylaxis infusions administered during the study were associated with a less than expected therapeutic effect (LETE) occurrence, which was defined as a spontaneous bleed occurring within 48 hours of a prophylaxis infusion. The majority (82.1%) of bleeding episodes in the prophylaxis arm were resolved after one infusion.

Adverse events observed in the study, for both the prophylaxis and on-demand periods, were consistent with the known adverse event profile of BeneFIX. The most common adverse events reported during the prophylaxis treatment period were arthralgia (20%), upper respiratory infection (20%), toothache (20%), pyrexia (16%), headache (16%), pharyngitis (12%), back pain (12%) and local swelling (12%). No inhibitor development, thrombotic events or allergic reactions related to this product were observed in this study.

These results are preliminary, top-line data and are subject to additional analyses. Complete results from this study will be submitted for presentation at upcoming medical congresses and submitted for publication in a peer-reviewed journal.

Study Background

This study was a Phase 3, open-label, non-randomized two-period study consisting of six months of on-demand therapy only, followed by 12 months of routine prophylaxis with BeneFIX 100 IU/kg once-weekly. Participants included in this study were males with a mean age of 31.3 years (N=25) and moderately severe to severe hemophilia B (FIX:C ≤2%). Of the 25 participants enrolled in the study, all received at least one dose of study drug, and no participants discontinued treatment early. The mean duration of treatment was 550 days.

About BeneFIX

BeneFIX is a recombinant coagulation factor IX product indicated for the control, prevention and perioperative management of bleeding episodes in adult and pediatric patients with hemophilia B. BeneFIX received FDA approval in the U.S. on February 11, 1997, and was approved in the European Union later that year. It has been studied in clinical trials in both previously treated and untreated patients, and established in both on-demand and preventive care, additionally shown to help control bleeds in major and minor surgeries. BeneFIX is not approved for prophylaxis use in the United States.

BeneFIX Indications and Usage

BeneFIX is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease.

BeneFIX is NOT used to treat hemophilia A.

Important Safety Information for BeneFIX

BeneFIX is contraindicated in patients who have manifested life-threatening, immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including hamster protein. Call your health care provider right away if your bleeding is not controlled after using BeneFIX. Allergic reactions may occur with BeneFIX. Call your health care provider or get emergency treatment right away if you have any of the following symptoms: wheezing, difficulty breathing, chest tightness, your lips and gums turning blue, fast heartbeat, facial swelling, faintness, rash or hives. Your body can make antibodies, called “inhibitors,” which may interfere with the effectiveness of BeneFIX. If you have risk factors for developing blood clots, such as a venous catheter through which BeneFIX is given by continuous infusion, BeneFIX may increase the risk of abnormal blood clots. The safety and efficacy of BeneFIX administration by continuous infusion have not been established. Some common side effects of BeneFIX are nausea, injection site reaction, injection site pain, headache, dizziness and rash.
Please see full Prescribing Information for BeneFIX available at www.BeneFIX.com.

About Hemophilia

Hemophilia is a type of bleeding disorder that causes the blood to take a long time to clot as a result of a deficiency in one of several blood clotting factors, and occurs almost exclusively in males. People with hemophilia B have a deficiency in clotting factor IX, a specific protein in the blood. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. People with hemophilia face specific risks and need to be careful not

to cause injury to their bodies, as injuries can prompt a bleed, which have the potential to be life threatening.

Pfizer Inc.: Working together for a healthier world®

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. To learn more, please visit us at www.pfizer.com.

Media: Jennifer Kokell, (212) 733-2596 Investor: Ryan Crowe, (212) 733-8160