Pfizer Logo

Pfizer Announces Publication of Study Results of BeneFIX® Coagulation Factor IX (Recombinant) Once-Weekly Prophylaxis for Hemophilia B

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Phase 3 Study showed Prophylaxis With BeneFIX Significantly Reduced Annualized Bleeding Rate Compared to On-Demand Therapy

Pfizer Inc. (NYSE:PFE) today announced the publication of the Phase 3 study results of a once-weekly regimen of BeneFIX® Coagulation Factor IX (recombinant) 100 IU/kg prophylaxis versus on-demand treatment in people with moderately severe or severe hemophilia B. The findings were published in*Haemophilia,*1 the official journal of the World Federation of Hemophilia.

“In this study, a once-weekly prophylactic regimen with BeneFIX provided a significant reduction in bleeding, which is an important goal in managing hemophilia,” said Pablo Rendo, M.D., Global Clinical Lead, Senior Director, Physician Clinicians at Pfizer.

The study showed that the median ABR (annualized bleeding rate) values were 2.0 (range, 0.0-13.8) for the prophylaxis period and 33.6 (range, 6.1-69.0) for the on-demand period. The mean ABR values were 3.6 ± 4.6 for the prophylaxis period and 32.9 ±17.4 for the on-demand period, which were statistically significantly different ($p < 0.0001$).1 During the 52 weeks of the prophylaxis period, 36 percent of patients experienced no bleeding events of any kind, and 48 percent of patients experienced no spontaneous bleeding events.1

Almost half (47 percent) of the 17 factor IX activity levels measured approximately one week post-dose were greater than 2 IU/dL (2.1-10.4IU/dL), and three patients had zero ABR despite a factor IX activity level of zero.1

In the study, the adverse events reported for the prophylactic regimen were similar to those reported for on-demand treatment. The most common adverse events, occurring in 10 percent or more of patients during either regimen, were arthralgia, back pain, headache, joint swelling, local swelling, nasopharyngitis, pharyngitis, pyrexia, toothache and upper respiratory tract infection.1 Neither inhibitors nor thrombotic events were reported. 1

Study Background

This was a Phase 3, international, non-randomized, open-label sequential-period trial with a six-month period of on-demand treatment followed by 12-month period of prophylaxis, with a mean total therapy duration of 550 days.1 All 25 participants had moderately severe or severe hemophilia B (factor IX activity 2 IU/dL or less) and were male with a mean age of 31.3 years; participants had experienced at least 12 bleeding events, six of them in joints, in the previous year. 1 All participants received BeneFIX, and no one discontinued treatment early. 1

About BeneFIX

BeneFIX received first approval in the United States on February 11, 1997. In the United States, BeneFIX is indicated for the control and prevention of bleeding episodes in adult and pediatric patients with hemophilia B, including perioperative management. BeneFIX is not approved for prophylactic use in the United States.

BeneFIX is approved in Europe for treatment and prophylaxis of bleeding in patients with hemophilia B.

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 stop BeneFIX from working properly.
- 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 (link is external).

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 and Rare Diseases

Rare diseases are among the most serious of all illnesses and impact millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact in addressing unmet medical needs. The Pfizer focus on rare diseases builds on more than a decade of experience and a global portfolio of more than 20 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders, pulmonology, and oncology.

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1 Kavakli K, Smith L, Kuliczkowski K, et al. Once-weekly prophylactic treatment versus on-demand treatment with nonacog alfa in patients with moderately severe to severe hemophilia B. *Haemophilia*. 2016; Jan 29. doi: 10.1111/hae.12878. [Epub ahead of print].

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