FDA News Release

FDA approves new treatment to prevent bleeding in certain patients with hemophilia A

For Immediate Release

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Summary

FDA approves new treatment to prevent or reduce frequency of bleeding episodes in patients with hemophilia A who have Factor VIII inhibitors.

Release

The U.S. Food and Drug Administration today approved Hemlibra (emicizumab-kxwh) to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients with hemophilia A who have developed antibodies called Factor VIII (FVIII) inhibitors.

“Reducing the frequency or preventing bleeding episodes is an important part of disease management for patients with hemophilia. Today’s approval provides a new preventative treatment that has been shown to significantly reduce the number of bleeding episodes in patients with hemophilia A with Factor VIII inhibitors,” said Richard Pazdur, M.D., acting director of the Office of Hematology and Oncology Products in the FDA’s Center for Drug Evaluation and Research and director of the FDA’s Oncology Center of Excellence. “In addition, patients treated with Hemlibra reported an improvement in their physical functioning.”

Hemophilia A is an inherited blood-clotting disorder that primarily affects males. According to the National Institutes of Health, hemophilia affects one in every 5,000 males born in the United States, approximately 80 percent of whom have hemophilia A. Patients with hemophilia A are missing a gene which produces Factor VIII, a protein that enables blood to clot. Patients may experience repeated episodes of serious bleeding, primarily into their joints, which can be severely damaged as a result. Some patients develop an immune response known as a FVIII inhibitor or antibody. The antibody interferes with the effectiveness of currently available treatments for hemophilia.

Hemlibra is a first-in-class therapy that works by bridging other Factors in the blood to restore blood clotting for these patients. Hemlibra is a preventative (prophylactic) treatment given weekly via injection under the skin (subcutaneous).

The safety and efficacy of Hemlibra was based on data from two clinical trials. The first was a trial that included 109 males aged 12 and older with hemophilia A with FVIII inhibitors. The randomized portion of the trial compared Hemlibra to no prophylactic treatment in 53 patients who were previously treated with on-demand therapy with a bypassing agent before enrolling in the trial. Patients taking Hemlibra

experienced approximately 2.9 treated bleeding episodes per year compared to approximately 23.3 treated bleeding episodes per year for patients who did not receive prophylactic treatment. This represents an 87 percent reduction in the rate of treated bleeds. The trial also included patient-reported Quality of Life metrics on physical health. Patients treated with Hemlibra reported an improvement in hemophilia-related symptoms (painful swellings and joint pain) and physical functioning (pain with movement and difficulty walking) compared to patients who did not receive prophylactic treatment.

The second trial was a single arm trial of 23 males under the age of 12 with hemophilia A with FVIII inhibitors. During the trial, 87 percent of the patients taking Hemlibra did not experience a bleeding episode that required treatment.

Common side effects of Hemlibra include injection site reactions, headache, and joint pain (arthralgia).

The labeling for Hemlibra contains a boxed warning to alert healthcare professionals and patients that severe blood clots (thrombotic microangiopathy and thromboembolism) have been observed in patients who were also given a rescue treatment (activated prothrombin complex concentrate) to treat bleeds for 24 hours or more while taking Hemlibra.

The FDA granted this application **Priority Review (/ForPatients/Approvals/Fast/ucm405405.htm)** and **Breakthrough Therapy (/ForPatients/Approvals/Fast/ucm405397.htm)** designations. Hemlibra also received **Orphan Drug (http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/default.htm)** designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The FDA granted the approval of Hemlibra to Genentech, Inc.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation’s food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

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Inquiries

Media

Angela Stark (mailto:angela.stark@fda.hhs.gov)
301-796-0397

Consumers

888-INFO-FDA

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- **FDA: Office of Hematology and Oncology Products (/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm091745.htm)**
- **FDA: Approved Drugs: Questions and Answers**

(/Drugs/ResourcesForYou/Consumers/ucm054420.htm)

- FDA: Fast Track, Breakthrough Therapy, Accelerated Approval, Priority Review (https://www.fda.gov/ForPatients/Approvals/Fast/default.htm)
- NIH: Hemophilia (https://www.nhlbi.nih.gov/health/health-topics/topics/hemophilia/)

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