FDA Approves Ferriprox to Treat Patients with Excess Iron in the Body

Oct 14, 2011, 15:10 ET from U.S. Food and Drug Administration

FDA Logo. (PRNewsFoto/U.S. Food and Drug Administration (FDA))

SILVER SPRING, Md., Oct. 14, 2011 /PRNewswire-USNewswire/ -- The U.S. Food and Drug Administration today approved Ferriprox (deferiprone) to treat patients with iron overload due to blood transfusions in patients with thalassemia, a genetic blood disorder that causes anemia, who had an inadequate response to prior chelation therapy.

(Logo: http://photos.prnewswire.com/prnh/20090824/FDALOGO)

Patients with thalassemia have excess iron in the body from the frequent blood transfusions (transfusional iron overload), a condition that is serious and can be fatal. These patients also have a risk of developing liver disease, diabetes, arthritis, heart failure or an abnormal heart rhythm.

The standard of care to treat transfusional iron overload is chelation therapy – chemical agents that are used to remove heavy metals from the body. Ferriprox is intended for use when chelation therapy is inadequate.

"Ferriprox represents the first new FDA-approved treatment for this disorder since 2005," said Richard Pazdur, M.D., director of the Office of Hematology and Oncology Products in the FDA's Center for Drug Evaluation and Research.

The safety and effectiveness of Ferriprox is based on an analysis of data from twelve clinical studies in 236 patients. Patients participating in the study did not respond to prior iron chelation therapy. Ferriprox was considered a successful treatment for patients who experienced at least a 20 percent decrease in serum ferritin, a protein that stores iron in the body for later use. Half of the patients in the study experienced at least a 20 percent decrease in ferritin levels.

The most common side effects seen in patients who received Ferriprox included nausea, vomiting, abdominal and joint pain, urine discoloration (chromaturia), a decrease in the number of white blood cells (neutropenia), and an increase in the level of a liver enzyme that may be indicative of tissue or liver damage at unsafe amounts.

The most serious side effect seen in about two percent of patients treated with Ferriprox was the development of agranulocytosis, a serious and potentially life-threatening reduction in the number of granulocytes (a type of white blood cell that fights infection).

The therapy is being approved under the FDA's accelerated approval program, designed to provide patients with earlier access to promising new drugs followed by further studies to confirm the drug's clinical benefit.

The accelerated approval program allows the agency to approve a drug to treat a serious disease based on clinical data showing that the drug has an effect on an endpoint that is reasonably likely to predict a clinical benefit to patients, or on an effect on a clinical endpoint other than survival or irreversible morbidity (illness).

ApoPharma has agreed to several post-marketing requirement and commitments. One commitment includes further study of the use of Ferriprox in patients with sickle cell disease who have transfusional iron overload.

Earlier this year, the U.S. Department of Health and Human Services (HHS) launched the Sickle Cell Disease (SCD) Initiative bringing together HHS agencies to enhance the quality and quantity of SCD data, develop best practice guidelines and quality of care metrics, improve health care delivery and coordination of care for patients with SCD, facilitate approval of new medical products, and expand research on SCD. The post-marketing requirement for further study of Ferriprox aligns with the goals of the SCD Initiative.

Ferriprox is marketed by ApoPharma Inc. of Toronto.

For more information:

FDA: Office of Hematology and Oncology Products
http://www.fda.gov/AboutFDA/CentersOffices/CDER/ucm091745.htm

FDA: Spotlight on Drug Innovation – Update of FDA's novel drug approvals in 2011
http://www.fda.gov/Drugs/InformationOnDrugs/ApprovedDrugs/ucm254242.htm

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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