DEPARTMENT OF HEALTH & HUMAN SERVICES

Food and Drug Administration
10903 New Hampshire Avenue
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Silver Spring, MD 20993

MAR 7 2013

Sidney M. Wolfe, M.D., Director
Michael Carome, M.D., Deputy Director
Public Citizen Health Research Group
1600 20th St., NW
Washington, D.C. 20009

Dear Drs. Wolfe and Carome,

Thank you for your letter of October 12, 2011 in which you express your opposition to approval of New Drug Application (NDA) 021825 for Ferriprox (deferiprone) for treatment of transfusional iron overload when current chelation is inadequate.

As you are aware, on Oct. 14, 2011 the Food and Drug Administration (FDA or the Agency) approved Ferriprox to treat iron overload due to chronic transfusions in patients with thalassemia who have not adequately responded to other chelators (deferoxamine).

The safety and effectiveness of Ferriprox is based on a prospective analysis of data from 236 patients from 12 clinical trials who failed to respond to available therapy. Ferriprox was considered a successful treatment by the Agency when patients receiving the drug experienced at least a 20 percent decrease in serum ferritin, a protein that stores iron in the body for later use. Fifty percent of the patients in the study experienced at least a 20 percent decrease in ferritin levels.

You refer to discussion among members of the FDA Oncologic Drugs Advisory Committee (ODAC) during the ODAC meeting on September 14, 2011 in which Ferriprox was considered. We acknowledge the Advisory Committee discussion was substantive, and that some Committee members noted reservations about a marketing approval for Ferriprox. As you are aware, we are not bound by Advisory Committee voting — however, we take all data and interpretations shared at Advisory Committee meetings under careful review.

Given the limited treatment options for patients who fail to respond to other chelation therapies, FDA approved Ferriprox under the Agency's accelerated approval program, a provision which allows us to approve a drug to treat a serious or life-threatening disease based on clinical data showing that the drug has an effect on an endpoint that is reasonably likely to predict a clinical benefit for patients.

The company, ApoPharma, is required to conduct additional clinical trials confirming this benefit to remain on the market and, additionally, ApoPharma has agreed to several post-marketing requirements, including the further study of Ferriprox use in patients with sickle cell disease who have transfusional iron overload. We expect additional data on Ferriprox according to the schedule contained in the approval letter (available online at

http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm?fuseaction=Search.Label_ApprovalHistory#apphist).

While we are certainly aware of the complex pathway to approval for Ferriprox that you describe at length in your letter, we would not agree that these sorts of challenges suggest that a drug product should not be approved. We are committed to working with companies in a manner that helps them address the significant clinical issues we identify in an application. Our primary goal as an Agency is to provide patients with access to innovative and promising new drug therapies that have demonstrated that they are reasonably safe and effective in the intended patient population.

We are aware that this reply has been delayed. We appreciate your understanding that the Agency must often manage competing priorities that can at times lead to delayed responses.

Thank you for your letter and for sharing your perspectives with us.

Sincerely,

Janet Woodcock, M.D.
Director
Center for Drug Evaluation and Research