

Genzyme Fabrazyme® - FDA asked to allocate full doses of Fabrazyme® to treat Fabry Disease Patients in the U.S. prior to exporting it overseas

Washington, DC – January 19, 2011, -- Today victims of the genetic illness Fabry Disease, (Joseph M. Carik of North Las Vegas, Nevada, and Amber Britton of Kirkland Washington) petitioned to allocate full doses of Fabrazyme® to U.S. Fabry patients. Fabrazyme® is manufactured by Genzyme Corporation and it is the only approved FDA enzyme replacement treatment for Fabry disease, a relatively rare genetic disease. Currently, no newly diagnosed patients such as Amber Britton are eligible for treatment and pre-existing patients such as Mr. Carik are rationed to less than half of the recommended dosage. As a result patients' symptoms have returned and patients are increasingly likely to die of the disease. The U.S. situation is dire with as many as three deaths of Fabry patients reported during the drug shortage.

Genzyme, which produces Fabrazyme® under an exclusive license from Mt. Sinai Medical Center, has been unable to produce enough drug to treat the US Fabry disease market since mid-2009 due to various manufacturing errors. As a result, Genzyme entered into a consent decree with the FDA in which Genzyme agreed to a fine of \$175 million dollars.

However, 62% of Fabrazyme® being manufactured in the U.S. is allocated to patients overseas, even though such patients have access to the alternative drug, Replagal® manufactured by Shire Pharmaceuticals. Since April of 2010, the European Medicines Agency has recommended that patients either receive a full dose of Fabrazyme® or be switched to Replagal®. As a result there is no drug shortage in Europe.

In the U.S., Replagal® is not an FDA approved drug, and Shire has withdrawn its application for FDA approval, so no alternatives exist. The U.S. is the only country in the world where Fabry patients receive no treatment for their disease, even though the discovery and patents were obtained as a result of public funding by the National Institutes of Health.

The petition filed with the FDA requests that Fabrazyme® be allocated first to U.S. patients and, if it is not, that the patients be allowed to present their case for medical need before it the drug is sent overseas. The FDA has allowed some Fabry patients to import Replagal® from Europe for emergency medical use in the U.S. However, patients must petition the FDA and then show a critical medical need.

The FDA has the authority to regulate distribution of drugs during a shortage under its consent decree with Genzyme®. It has previously exercised implemented an allocation program for repository corticotropin injection (Questcor Pharmaceuticals), caspofungin acetate (Cancidas, Merck) and beta methasone sodium phosphate (Celestone Soluspan, Schering).

A copy of the Citizen Petition is available on the web at <http://www.patentlawyersite.com/files/Download/Web%20CITIZEN%20PETITION%20TO%20ALLOCATE%20AGALSIDASE%20BETA.doc>

Mr. Carik previously petitioned HHS to allow manufacturers to produce Fabrazyme® under a Bayh-Dole march-in license. Ms. Britton formally supported the petition. However, the request was denied last month by the NIH stating that FDA regulations prevented another manufacturer from making the drug in a timely manner. Specifically, the NIH ruled that despite the critical health need, the FDA regulations would require manufacturers more than three years to obtain FDA approval. Thus, granting march-in rights to manufacturers would be futile.

Mr. Joseph M. Carik was diagnosed with Fabry disease in 2005, before the shortage so he receives only a 30% dose. Ms. Britton was diagnosed in 2010 after the shortage so she is not allowed access to the drug. As a result of rationing, all petitioners have had their symptoms return, including pain and burning in their extremities (neuropathy); decreased kidney function (proteinuria), severe gastrointestinal symptoms, and cardiac problems.

Fabry disease is a rare disorder with an estimated prevalence in the general population of 1 in 117,000 people. Those with the disease are unable to metabolize fats properly leading to numerous symptoms, the most serious of which are renal failure and degenerative heart disease. Most patients did not live much beyond 50 prior to the development of enzyme replacement therapy such as Fabrazyme®.

The petitioners are represented pro bono in the matter by C. Allen Black, Ph.D. who is a licensed patent attorney. Prior to attending law school he was an assistant professor at the University of Pittsburgh Medical School where he researched infectious diseases and vaccines. He currently is in private practice and teaches Biotechnology law at the University of Pittsburgh law school.

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