



Cooley's Anemia
FOUNDATION
Leading the Fight Against Thalassemia

PRESS RELEASE

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Cardiac Deaths Among Thalassemia Patients in the United States Preventable Treatment Option Awaits FDA Approval

Lake Buena Vista, FL - Overwhelming data showing protection of the heart in iron overloaded patients treated with the oral iron chelator deferiprone (*Ferriprox*TM) were presented at the Eighth Cooley's Anemia Symposium, sponsored by the New York Academy of Sciences and the Cooley's Anemia Foundation and held March 17-19, 2005 in Lake Buena Vista, Florida. These data, including a stunning report on the morbidity and mortality of Cooley's anemia patients in Italy, has fueled the Foundation's campaign to support the approval of deferiprone, a product approved in 47 countries, but not yet in the United States.

Cooley's anemia, also called thalassemia, is a fatal genetic blood disease, the treatment of which results in the toxic accumulation of iron, leading in turn to cardiac disease. In the U.S., the only currently approved treatment for iron overload is deferoxamine, a drug which must be administered nightly via pump for a 10-12 hour period.

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The study, presented by Dr. Caterina Borgna-Pignatti from Ferrara, Italy, involved more than 500 patients treated with deferiprone or deferoxamine during the past 9 years. Dr. Borgna-Pignatti reported on patients from 7 centers in Italy monitored for heart disease and mortality. In this retrospective study, when the patients who used deferiprone were compared to those who used only deferoxamine, it was found that heart disease and death were significantly more frequent in those who had not received deferiprone. This was true even though the deferiprone-treated group was more heavily iron loaded prior to starting the drug. The vast majority of the deaths on the deferoxamine-treated group were cardiac-related.

In subsequent presentations, Dr. Paul Telfer, from the U.K., commented that similar results were seen following the introduction of deferiprone in the U.K. in the fall of 1999 and that the death rate from cardiac disease in the U.K. since that time has dramatically declined compared to previous years. He also stated that in Cyprus, where there was a high percentage of deferiprone use, the death rate had plummeted as well.

Dr. Antonio Piga, from Torino in Italy, confirmed that in his own center, patients on deferiprone had a much lower prevalence of heart disease and death than those on deferoxamine.

Dr. Dudley Pennell, a cardiologist with Royal Brompton Hospital and Imperial College in London, provided scientific evidence that may explain the reason for the cardio-protective effects of deferiprone. He noted that in a retrospective study, deferiprone was far more effective than deferoxamine in reducing the levels of iron in the heart, based upon the new technique of magnetic resonance imaging (MRI) using the T2* approach to evaluate iron in the heart.

Pennell then reported on the results of the first randomized comparative prospective study, comparing deferiprone vs. deferoxamine and using MRI T2* to assess heart iron

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concentrations. He found that in just one year, deferiprone lowered iron in the heart much better than deferoxamine; more importantly, cardiac function (measured by left ventricular ejection fraction) was improved with deferiprone but not with deferoxamine. Pennell's work indicates that deferiprone, by efficiently removing iron from the heart, is able to have a cardio-protective effect even better than deferoxamine.

Dr. Sergio Piomelli, a member of the Foundation's Medical Advisory Board, commented that the U.S. must do all that it can to catch up with the state-of-the-art treatment of iron overload in thalassemia. He pointed out that in Europe, where patients have been using deferiprone for years, the death rate has dropped drastically; in the U.S., a high mortality rate continues to plague thalassemia patients.

Frank Somma, National President of the Cooley's Anemia Foundation also addressed the number of U.S. patients who have passed away from Cooley's anemia over the past year and called all physicians into action, endorsing a petition to the Federal Food and Drug Administration urging the agency to act swiftly and favorably for the approval of treatment alternatives such as deferiprone.

About the Cooley's Anemia Foundation and Cooley's anemia/thalassemia

Founded in 1954, the Cooley's Anemia Foundation is dedicated to serving people afflicted with various forms of thalassemia, most notably the major form of this genetic blood disease, Cooley's anemia/thalassemia major. The Foundation's mission is advancing the treatment and cure for this fatal blood disease, enhancing the quality of life of patients and educating the medical profession, trait carriers and the public about Cooley's anemia/thalassemia.

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In its most severe form, thalassemia affects the body's ability to form red blood cells that can effectively carry oxygen to various parts of the body, thus requiring red blood cell transfusions as often as every two weeks. While sustaining life, these transfusions result in the toxic accumulation of iron in the body. Cardiac disease as a result of heart iron accumulation is the most prevalent cause of death among patients with thalassemia.

For patients in the United States there is currently only one treatment option to remove iron: deferoxamine, a drug which must be subcutaneously administered nightly by pump over a 10-12 hour period.

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