

## Safety of Deferasirox (ICL670) and Deferoxamine (Desferal or DFO) Combined Chelation Therapy in Patients with Transfusion Dependent Thalassemia and Iron Overload

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### Synopsis:

Iron overload is caused by blood transfusions. Patients with thalassemia and other inherited anemias must have transfusions to treat their disease. Because the human body is not able to get rid of the iron coming from the transfused blood, it builds up in some organs such as the liver and heart, causing complications. Complications may include heart failure, irregular heart rhythms or beating, in addition to diabetes mellitus, growth delay and decreased metabolism related to hormone deficiencies, among others. The treatment needed to remove the excess iron from the body is called iron chelation therapy. This therapy may stabilize or prevent the complications from happening.

Over the last 30 years, we have known that the FDA approved drug deferoxamine (Desferal) has helped as an iron chelator by taking iron out of the liver. It must be given with a needle over 12-24 hours (by an infusion pump). A new iron chelator, ICL670 (Exjade), is now available. Exjade is in pill form and is taken by dissolving the pill into a glass of water and then drinking it once the pill has dissolved. As of January, 2006, Exjade was approved for clinical use by the FDA in the United States

Patients with iron overload are starting to be given a combination of Desferal and Exjade clinically because both drugs are FDA approved. Since these drugs have not been in combination, the researchers in this study want to follow patients getting this drug combination very closely for one year to make sure all safety issues are explored fully.

Fifteen people will be enrolled in this study, 5 in each of the 3 study groups. Patients will all be enrolled at Children's Hospital & Research Center at Oakland (CHRCO) but may live outside our service area. All of the patients living remote to CHRCO, will be followed clinically by a doctor who is familiar with the research protocol.