

**Title: REPLAGAL (agalsidase alfa) for Emergency Use in Fabry Disease**

**IRB# 2010-004**

**Principal Investigator: Paul Harmatz, MD**

**Synopsis:**

In light of the ongoing manufacturing problems with FABRAZYME, the sponsor has agreed to provide REPLAGAL through a compassionate use program supported by the FDA. One adult subject will be enrolled at CHRCO. The enzyme therapy REPLAGAL (agalsidase alfa) is approved for treatment of Fabry disease in Europe and Canada. Both REPLAGAL and FABRAZYMW have demonstrated efficacy in human clinical trials, but different endpoints and dosing regimens used in trials prevent direct comparison of the relative efficacies of the two products. Shire HGT will provide enzyme for up to 6 months under this emergency IND. No data collection is planned. This intervention is necessary due to the patient's declining renal function, continuing shortage of FABRAZYME, and lack of other approved therapeutic alternatives.