Genzyme has announced a drug shortage for Fabrazyme, the only enzyme replacement therapy (ERT) approved in the US for Fabry disease. Genzyme has established the following rationing system for Fabrazyme in response to the drug shortage:

1. Fabry patients under 18 years of age will continue to receive treatment as scheduled.
2. Adult Fabry patients (both male and female) who are stable on Fabrazyme will receive a 70% reduction in dose (e.g., from 1gm/kg/dose, biweekly to 0.3 gm/kg/dose, biweekly).

The shortage of Fabrazyme is expected to last until the end of 2009. As a result of this shortage, patients with Fabry disease have submitted inquiries to FDA about access to other treatment options, including Replagal, another enzyme replacement therapy manufactured by Shire Human Genetic Therapies, Inc. Replagal has not been approved in the U.S., but has been approved in other countries for the treatment of Fabry disease. In response to the shortage of Fabrazyme, FDA has been in discussion with Shire regarding possible options that would allow Fabry patients in the U.S. access to Replagal.

At this time, individual Fabry patients may access treatment with Replagal under emergency or single-patient INDs based on clinical need as assessed by their treating physician. Treating physicians interested in pursuing emergency or single-patient INDs for their patients must first contact Shire to obtain agreement from the firm that they will supply Replagal. Once agreement from Shire is obtained, treating physicians may then contact FDA to request the IND.

Please contact LCDR Chantal Phillips (chantal.phillips@fda.hhs.gov), Division of Gastroenterology Products, for further details.