

## MTAP Fact Sheet June 2007

MTAP, the Myozyme® (alglucosidase alfa) Temporary Access Program, is a clinical program to provide access to Myozyme produced by a larger scale manufacturing process for a limited time until this manufacturing scale is approved by the FDA. Patients enrolled in MTAP will be provided Myozyme at no cost during the course of the program. The goals of MTAP are two-fold: (a) to minimize any potential disruption in the treatment of patients already on therapy; and (b) to ensure that Myozyme is available for new patients who are in the most urgent clinical need.

### **General Information**

- **Beginning immediately, new patients 18 years of age and older who meet either of the following criteria will be eligible for enrollment into the Myozyme Temporary Access Program: (1) require ventilation assistance for breathing, or (2) require a wheelchair for ambulation.**
- For new patients who do not meet the MTAP entry criteria, Myozyme treatment will be delayed until the larger scale manufacturing process is approved for commercial use.
- Genzyme will invite some patients who are currently on treatment and are 18 years of age or older to enroll into MTAP to optimize supply availability. Genzyme will contact physicians directly regarding the transition for patients currently on treatment.
- The program will require IRB approval at treatment sites, and like all clinical programs, patients will be required to sign an informed consent to enroll into MTAP once their treating site has received IRB approval.
- Genzyme anticipates an FDA decision in the fourth quarter of 2007. Following the approval of the larger scale manufacturing process, we will help all patients enrolled in MTAP and new patients waiting to initiate treatment expeditiously transition to commercial therapy.
- Physicians seeking additional clarification or information should contact Genzyme Medical Information at 1-800-745-4447, option 2. Patients should contact Genzyme Treatment Support at 1-800-745-4447, option 3.