

MTAP Fact Sheet July 2007

MTAP, the Myozyme® (alglucosidase alfa) Temporary Access Program, is the primary mechanism to help ensure continued access to treatment in the United States. It is a clinical program to provide Myozyme produced by a larger scale manufacturing process for a limited time until this manufacturing scale is approved for use in the United States 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 the disruption in the treatment of patients already on therapy; and (b) to help ensure that Myozyme is available for new patients who are in the most urgent clinical need.

General Information

- **Beginning immediately, patients 18 years of age and older who are currently on commercial treatment are strongly urged to seek enrollment into MTAP.**
- New patients 18 years of age and older who meet either of the following criteria will also 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 contact physicians directly regarding the transition of patients currently on treatment and patients who are 18 years of age and older should immediately contact their treating physician to discuss enrollment in MTAP.
- MTAP requires Institutional Review Board (IRB) approval at each treatment site which may be a lengthy process, thus, immediate action is paramount. Patients will be required to sign an informed consent to enroll into MTAP. Adults currently on therapy who do not enroll into MTAP can not be assured of continued access to treatment.
- Genzyme anticipates an FDA decision in the first quarter of 2008 at the earliest. 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.