

October Update on the US Myozyme[®] Supply

Status Update on FDA Submission

Genzyme will resubmit the application for the larger scale manufacturing process of Myozyme[®] (alglucosidase alfa) to the FDA by the end of the year. We continue to anticipate an FDA decision sometime during the first quarter of next year at the earliest. Genzyme and the FDA continue to work collaboratively and productively on managing the current US supply situation.

MTAP Transition

Genzyme continues to work closely with treating physicians to enroll all adult patients in the United States into MTAP (Myozyme Temporary Access Program). It is imperative that all physicians and adult patients continue to work with Genzyme to move through this transition in a timely manner. **During this resubmission period and pending an FDA decision on the larger scale manufacturing process, it is extremely important to continue to enroll as many adults into MTAP as possible to effectively manage the current limited US supply of Myozyme and prevent a stock out.**

There are currently 33 sites approved for MTAP, and many others are in the process of receiving IRB approval. Genzyme has created an MTAP website, http://www.myozyme.com/MTAP/mtap_pt.asp to help keep the patient and physician communities updated on the status of MTAP. The website is updated weekly. The site contains important information including:

- Contact information for Genzyme and the US Pompe Patient Associations
- Communications related to MTAP
- List of all approved MTAP sites
- List of sites that are in the process of being approved and are expected to treat patients upon approval
- Sites that are willing to treat patients who are not yet enrolled in MTAP but are willing to travel to a new site temporarily

Missed Infusion Update

Genzyme is greatly appreciative of the participation from the Pompe patient and physician community to date in the voluntary missed infusions for August and September/October. While we recognize that volunteering to miss an infusion is a difficult decision, adult participation in this activity has helped Genzyme to manage the commercial supply during this period. **We are no longer asking children 17 years of age and under to participate in this measure, but we continue to ask all adults who are not yet enrolled into MTAP and have not yet voluntarily missed an infusion to plan to miss an infusion in October if they have not already done so.** Please contact your physician directly to plan to miss an infusion in October.

Children 17 and under

Genzyme would like to clarify how the US Myozyme supply shortage may impact those who are 17 years of age and under and currently unable to enroll into MTAP. MTAP remains a program

designed for adult participation. Genzyme continues to feel confident that by enrolling close to 100% of adults into this program, we will have enough reserve supply to meet the treatment needs of those who are 17 years of age and under. Genzyme continues to work with the FDA to discuss contingency planning in the event of an actual drug shortage to determine how to ensure continued access to treatment for children and infants.

Additionally, we are no longer asking any children who are 17 years of age and under to voluntarily miss any additional infusions. If you have already missed an infusion, we appreciate your participation, and it is not necessary to plan to miss any additional infusions at this time. If you have not voluntarily missed an infusion, you should not plan to miss one at this time.

In Closing

As Genzyme continues to closely monitor Myozyme inventory levels in the United States, we will continue to keep the patient community informed of the US Myozyme supply situation. We appreciate the ongoing patience and understanding that patients, their families, and their physicians have continued to demonstrate during this period of supply challenges.