

## US Myozyme<sup>®</sup> Supply Update July 25, 2007

As previously communicated in April, a standard part of the biologics drug development process is to incrementally scale-up manufacturing capacity as demand for the product increases. During the development of Myozyme<sup>®</sup> (alglucosidase alfa), Genzyme began the manufacturing process using a smaller scale, and has since scaled-up to produce larger quantities of product for commercial use worldwide. This larger scale manufacturing process is currently approved in 29 countries but not in the United States. In June 2007, Genzyme submitted documentation to the FDA for the licensure of the larger scale manufacturing process for Myozyme.

In recent discussions with the FDA related to our application, we were asked to provide additional information to help the FDA in their evaluation, which is a common request during regulatory reviews. The requested data are available and are being prepared for re-submission of this application. Unfortunately, this will extend the timeline for approval and we now anticipate an FDA decision in the first quarter of 2008 at the earliest.

Given this new timeline, even with the commercial smaller scale manufacturing process operating at maximum capacity, we are regrettably unable to continuously supply the US Pompe patient population with Myozyme. Therefore, in partnership with the patient and physician communities, we must now accelerate our efforts to manage the US Myozyme supply until FDA's approval of the larger scale manufacturing process.

***In order to minimize the impact to the patient community at large, Genzyme has determined that the following steps must be urgently taken:***

- Patients 18 years of age and older who are currently on commercial treatment are strongly urged to seek enrollment into the Myozyme Temporary Access Program (MTAP)

**AND**

- All patients who are not enrolled in MTAP, with the exception of infants, should plan to miss an infusion in the month of August, and one additional infusion in either September or October

Physician support and participation is extremely critical to help ensure the continued access to treatment for their patients. Patients should immediately discuss these steps with their treating physicians.

MTAP is now the primary mechanism to ensure continued access to treatment. We need to enroll close to 100% of the adult population into MTAP by early October to effectively manage the commercial supply of Myozyme in the US. Adults enrolling into MTAP receive treatment via a clinical program thus conserving the remaining commercial supply for those who are 17 years of age and younger.

**For children 17 years of age and younger, other than infants, who are currently on treatment:**

- The decision to miss infusions will be determined between parents and the treating physician based on the child's medical status.
- Parents and physicians should plan to miss this infusion in the month of August and one additional infusion in either September or October.

**For those 18 years of age and older who are currently on treatment:**

- Strongly urged to seek immediate enrollment into MTAP to ensure continued access to treatment. Once enrolled in MTAP, you will not be subject to missing future infusions as described below.
- Those not enrolled into MTAP as of August 1<sup>st</sup> should work with their physician to plan to miss one Myozyme infusion in August and an additional infusion in either September or October if still not enrolled in MTAP at that time. This will include patients who are attempting to enroll into MTAP but whose enrollment has not been completed for any reason.
- Those adults who do not enroll into MTAP cannot be assured of continued access to treatment. Because the MTAP enrollment process takes time, immediate action is paramount.

Patients should contact their treating physician or Genzyme Treatment Support Case Manager with questions and physicians should contact Genzyme Medical Information. Genzyme will continue to actively monitor the commercial smaller scale US Supply of Myozyme throughout this period, and will provide regular updates to the Pompe community.

We at Genzyme deeply regret the impact this situation is having on patients and their families. We will continue to work with the patient and physician community as well as the FDA to manage the Myozyme supply, and are appreciative of the ongoing support and understanding during this difficult time.