Genzyme Pompe Program Update- November 17, 2008

We would like to take this opportunity to provide an important update to the Pompe Community.

The US Food and Drug Administration (FDA) has informed Genzyme that the FDA plans an accelerated approval for alglucosidase alfa produced at the 2000 L bioreactor scale for the treatment of late onset Pompe disease. Prior to approval, the company and the agency first need to agree on the design of a post-approval verification study and the FDA must complete its review of the Risk Evaluation and Mitigation Strategy (REMS), which Genzyme submitted earlier this month. This REMS program is intended to ensure that distribution of the 2000 L product is prescribed and administered to the intended patient population. The FDA has classified this submission as a major amendment to the Biologics License Application (BLA) for alglucosidase alfa produced at the 2000 L scale. **Therefore, to provide time to agree on the design of a post-approval verification study and complete the review of the REMS submission, the FDA has changed the Prescription Drug User Fee Action (PDUFA) date to February 28, 2009.** Genzyme will be required to submit the final protocol for the verification study after approval.

Genzyme is pleased to have clarity from the FDA on what it will take for alglucosidase alfa produced at the 2000 L to be approved under an accelerated approval. An accelerated approval is not a priority or fast-track review. An accelerated approval is an approval for commercial marketing, but with the requirement to conduct further studies to verify clinical benefit. We will work closely with the FDA to design a post-approval verification study.

Due to our continued state of limited supply, regrettably, MTAP will remain closed to new adult patients throughout this 3 month extension. Upon approval of alglucosidase alfa produced at the 2000 L scale, it is anticipated that new adult patients with late-onset Pompe disease will be able to access product through regular commercial channels. Patients currently enrolled in MTAP can continue in MTAP until the 2000 L scale is approved, at which time we will work to transition MTAP participants to commercial therapy. Alglucosidase alfa produced at the 160 L scale continues to be commercially available for all patients 17 years of age and younger.

Genzyme recognizes the difficulty this delay may cause for some Pompe patients and their families, as well as the physicians managing their care and appreciate your ongoing patience and understanding.