

Genzyme Program Update November 10th, 2008

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

The US Food and Drug Administration (FDA) held a meeting of its Endocrinologic and Metabolic Drugs Advisory Committee on October 21, 2008 to discuss Genzyme's Biologics License Application (BLA) for alglucosidase alfa produced at the 2000 L bioreactor scale to treat late-onset Pompe disease in the United States. The Committee voted 16-1 that the Late Onset Treatment Study (LOTS) established the effectiveness of alglucosidase alfa produced at the 2000 L scale for the treatment of patients with late-onset disease. The majority of the committee members recommended an 'Accelerated Approval', which would allow the 2000L to be approved but with the requirement to conduct a verification study of clinical benefit of therapy with alglucosidase alfa. The committee also recommended that treatment with the 2000 L product should be made available to all patients with late-onset disease, without restrictions based on age.

The Advisory Panel vote is not an approval of the BLA and the FDA is not obligated to follow the panel's recommendations. Genzyme has received a Prescription Drug User Fee Action (PDUFA) date from the FDA of November 29, 2008 and anticipates a response from the FDA regarding the approval status of the BLA on or prior to this date. Until Genzyme receives the FDA's official response, we cannot speculate on the specific details of the approval status of the product produced at the 2000 L scale including the timing of when it will become commercially available, or the exact wording of the indication.

In the meantime, we would like to reiterate the current status of the product supply in the United States. Alglucosidase alfa produced at the 160 L scale continues to be available commercially for all patients 17 years of age and younger. The MTAP program remains closed to new patients at this time. Genzyme will update the community following the official response from the FDA.

Our commitment to provide treatment to all those in need remains unchanged and Genzyme appreciates the support and understanding the Pompe community has demonstrated during this process.