Pompe Program Update – May 22, 2009

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

Background

In February 2009, Genzyme received a Complete Response letter from the FDA in regards to the Biologics License Application (BLA) for alglucosidase alfa produced at the 2000L scale which will be called Lumizyme™ in the US. In the letter, the FDA outlined the items that needed to be addressed before approval can be granted.

Update

Genzyme has submitted the final documentation to address all items in the FDA's complete response letter for the company's application for Lumizyme.

Included in the submission are clinical data from Genzyme's Pompe Registry, the Risk Evaluation and Mitigation Strategy (REMS) details and the final labeling. The FDA has agreed that a verification study is no longer necessary. Additionally, Genzyme has completed all measures required to respond to the FDA warning letter regarding the company's Allston manufacturing facility. The FDA previously informed Genzyme that it would accept the complete response submission and initiate the review without requiring an inspection to occur first. Genzyme anticipates that its filing will be designated as a class 2 resubmission with a six month review period.

Genzyme and the FDA are also in active discussions regarding the submission of an application for the 4000 L-scale manufacturing process and are working collaboratively to determine the quickest path towards approval. Genzyme anticipates filing this submission later this quarter.

In the meantime, we would like to reiterate the current status of product supply in the United States. Myozyme® (alglucosidase alfa) produced at the 160 L scale continues to be available commercially for all patients 17 years of age and younger. At this time, treatment remains unavailable for new patients 18 and older and enrollment in MTAP remains closed.

Genzyme recognizes the difficulty this additional delay may cause for Pompe patients, their families and the physicians managing their care. Please know that we fully appreciate the consequences to the Pompe Community and continue to work diligently with the FDA, physicians, patient organization leaders and the greater community through this challenging period. We will provide further updates to the community as more information becomes available.