US Pompe Program Update - November 16, 2009

Genzyme would like to provide an update to the US Pompe community about the status of the application for Lumizyme™ (alglucosidase alfa) produced at the 2000 L scale in the United States.

On Friday, November 13, 2009, Genzyme received a complete response letter from the Food and Drug Administration (FDA) on the Lumizyme application. A complete response letter is issued when the review of an application is complete, but there are remaining concerns that preclude the FDA from approving the application. It summarizes the outstanding issues that need to be resolved and identifies actions necessary for the application to gain approval. In its letter, the agency stated that satisfactory resolution of deficiencies related to the Allston Landing manufacturing plant are required before the application for Lumizyme at the 2000 liter bioreactor scale can be approved. Genzyme believes the other elements of the Lumizyme review, such as the Risk Evaluation and Mitigation Strategy (REMS), the product label, and post-marketing requirements, have been satisfactorily addressed.

After making an enormous effort for more than two years in an attempt to make Lumizyme broadly available in the United States, we are deeply disappointed by this further delay. We intend to continue working with the FDA to address the outstanding issues identified in the complete response letter.

Several months ago, we notified the community that we would no longer be manufacturing alglucosidase alfa at the 2000 L scale as all six of the bioreactors in our Allston manufacturing facility are needed for the production of Fabrazyme® (agalsidase beta) and Cerezyme® (imiglucerase for injection). Genzyme has a new facility operating in Belgium with larger scale 4000 L bioreactors to help provide a long term, sustainable supply for the global Pompe community. This facility is now the primary manufacturing source of alglucosidase alfa for the majority of Pompe patients worldwide. Approval of the 4000 L process is necessary to provide adequate supply and full access to patients in the United States. Genzyme plans to work with the FDA to determine the most appropriate route to approval of product manufactured at the 4000 L scale in light of the complete response.

In the United States, the Alglucosidase Alfa Temporary Access Program (ATAP) program has been providing access to therapy for a limited number of adults (patients ≥ 18 years of age). For continued access to treatment, Genzyme amended the ATAP protocol in July to allow for the use of clinical product manufactured at the 4000 L scale. All sites enrolled in ATAP have transitioned or are in the process of transitioning to product produced at the 4000 L scale. Adult Pompe patients currently enrolled in ATAP will continue in the program until further notice. The ATAP program remains closed to new patients.

Genzyme continues to produce Myozyme® (alglucosidase alfa) at the 160 L scale which is reserved for patients 17 years and younger.

We will continue to keep you informed of relevant updates as information becomes available. In the meantime, please contact your physician, Genzyme case manager, local Genzyme representative, or Genzyme Medical Information at 1-800-745-4447, option 2 with any questions you may have. We appreciate your ongoing support as we continue to work to provide broader access to treatment for the full Pompe community.