genzyme

Pompe Program Update
March 2, 2009

Supply Update

In January 2009, Genzyme notified the Pompe community that we were experiencing a temporary constraint in the global supply of Myozyme® (alglucosidase alfa) produced at the 2000 L bioreactor scale and that inventories were extremely tight. We are pleased to inform you that the European Commission has approved the larger 4000 L scale production of Myozyme at Genzyme's manufacturing facility in Belgium for use within the European Union. With this approval, the demand on the 2000 L inventories that currently supply the rest of the world, including MTAP in the US, has been relieved and the supply constraint is now resolved. The larger-scale Belgium facility will be able to help meet the long-term needs of patients for Myozyme upon receipt of approval by regulatory agencies throughout the world.

Furthermore, as a result of this approval in Europe, the guidance issued to conserve Myozyme for infants and children is no longer in effect and all adult patients worldwide can immediately resume regular infusion schedules and physicians outside of the United States (US) are now able to initiate therapy for new adult patients. In addition, due to the high degree of participation throughout the world of adult patients who transitioned to monthly infusions in January and February, we would like to inform the community that Genzyme has not been made aware of any infants or children who missed infusions during this period. Please know that we at Genzyme deeply appreciate your support, engagement, and understanding in managing through this temporary global supply constraint.

FDA Decision on Lumizyme™ (alglucosidase alfa)

There is also an important update regarding our application for alglucosidase alfa produced at the 2000 L scale in the United States, which will be known as Lumizyme. The FDA was unable to approve Lumizyme on February 28th and has outlined, in a complete response letter we received on February 27th, the items that need to be addressed before approval can be granted. Genzyme and the agency had made substantial progress toward finalizing these items but were not able to complete them before the action date. Specifically, Genzyme and the FDA must agree on a final design for a verification study to confirm the clinical benefit of alglucosidase alfa produced at the 2000 L scale and on the details of a Risk Evaluation and Mitigation Strategy (REMS) for the product. Genzyme must also address some issues detailed in a separate warning letter that was also received on February 27th and related to observations made by the FDA during an inspection of our Allston Landing, Massachusetts manufacturing facility before approval can be granted. We are confident that all of the information can quickly be assembled and provided to the FDA. We will be communicating with the FDA this week to start resolving the outstanding issues and will submit a response as soon as possible.

Adult patients in the US currently enrolled in MTAP can continue to receive treatment via this access program and should return to regular bi-weekly infusion schedules now that the supply constraint on the 2000 L product has been resolved. Due to the supply capacity limitations of Myozyme produced at the 160 L scale, Myozyme in the US will continue to be commercially available only to patients 17 years of age and younger. Upon approval of Lumizyme in the US, it is anticipated that new adult patients with late-onset Pompe disease will be able to access product through regular commercial channels; however, at this time, enrollment in MTAP remains closed to new adult patients.

Although we are disappointed by this additional delay in the approval process, Genzyme now has further clarity from the FDA on which issues remain outstanding. Our commitment to finding a sustainable long-term solution for all those in need of therapy remains unchanged and we appreciate the support and understanding the US community has demonstrated during this lengthy process. We will continue to provide updates to the community as information becomes available.