genzyme
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Community Update for Pompe Families in the United States who Utilize Myozyme®
(alglucosidase alfa) Produced at the 160 L Manufacturing Scale

As you are aware, for the past several years Myozyme manufactured at the 160 liter (L) bioreactor scale has been closely managed and monitored as part of our efforts to preserve product for infants and children as Genzyme has a limited ability to build inventory at this smaller manufacturing scale. This product is currently utilized by approximately 100 patients in the United States only. At this time, the unexpected large number of older patients still using Myozyme and the growth of younger patients as they age, combined with a recent loss of 160 L material during the manufacturing process, has changed our expectations of available 160 L product supply.

We are now taking steps to help ensure an uninterrupted course of therapy for patients currently receiving Myozyme in the United States and to enable a long term sustainable solution for the constrained 160 L supply. To do this, we are proactively enacting a clinical program, the ADVANCE study, for patients using Myozyme in the US. The objective of this open-label study is to demonstrate efficacy and safety of treatment with alglucosidase alfa produced at the 4000 L scale in patients previously treated with 160 L scale alglucosidase alfa in the commercial setting. **Beginning on March 30, 2012, Genzyme will cease shipments of Myozyme 160 L to any US patient 12 months of age and older who is eligible to enroll in the ADVANCE study; US patients 12 months of age and older who are using Myozyme must be enrolled into the ADVANCE study to continue receiving therapy.** The following information is intended to help parents of young patients and patients themselves understand how they may be affected.

- ***Individuals 12 months of age and older who are currently using Myozyme in the United States will need to work with the treating physician to transition to the ADVANCE study prior to March 30, 2012 to continue to have uninterrupted access to therapy.*** Genzyme will be working with families and treating physicians to transition these individuals to the ADVANCE study to help ensure uninterrupted access to therapy produced at the 4000 L manufacturing scale.

- Individuals who are under 12 months of age and currently using Myozyme will continue to receive Myozyme commercially until they reach 12 months of age, at which time they will need to enroll into the ADVANCE study.

- Newly diagnosed patients in the United States who are not yet on therapy and are under 12 months of age can access treatment with Myozyme commercially as we will work to conserve the limited 160 L supply specifically for all patients under 12 months of age.

- Newly diagnosed patients in the United States who are not yet on therapy and are 12 months of age and older will be reviewed on a case by case basis by Genzyme Medical Affairs and, if eligible, may enroll in the ADVANCE study unless it is determined that the patient is eligible to receive commercial Lumizyme® (alglucosidase alfa).

- ***Individuals receiving therapy outside of the United States and individuals in the United States who are treated with Lumizyme will not experience any changes to their treatment regimen.*** It is only in the United States that Genzyme has 2 products available for the treatment of Pompe disease. All Pompe patients outside of the United States receive Myozyme produced at the 4000 L manufacturing scale and those individuals in the US who are prescribed Lumizyme also receive material produced at the 4000 L scale.

Conducting this study has three objectives:

1. To provide uninterrupted access to therapy for patients in the United States who currently use Myozyme
2. To preserve our limited inventory of Myozyme (160 L) for patients under 12 months of age and future infants in the US with Pompe who need immediate access to treatment
3. To generate clinical data of the 4000 L scale in patients 12 months of age and older in the United States who have been previously treated with Myozyme (160 L)

More information will be forthcoming; however, we know that you will likely have questions. Genzyme plans to host a US Pompe Community Town Hall at 7pm Eastern Time on Tuesday January 31st. Please contact your Genzyme case manager at 1-800-745-4447 option 3, the AMDA www.amda-pompe.org or the United Pompe Foundation www.unitedpompe.com for call details. In the meantime, please refer to your treating physician or your local Genzyme contact for additional information. Thank you.