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United States Pompe Community Update - October 17, 2014

Genzyme would like to share with you an important update regarding the recent United States (U.S.) Food and Drug Administration's (FDA) approval of an expanded label for Lumizyme® (alglucosidase alfa) manufactured at the 4000L scale and its impact on Myozyme® (alglucosidase alfa) manufactured at the 160L scale.

In our previous communication dated August 1, 2014, we informed you that the U.S. Food and Drug Administration (FDA) approved the supplement to expand the indication for Lumizyme. Lumizyme is now indicated for patients with Pompe Disease with no limitation as to age or phenotype. This approval was based on data that included biochemical analysis as well as clinical data from an investigator sponsored trial in Taiwan in infantile-onset patients. In addition, supportive data from the ADVANCE trial consisting of patients 12 months and older previously treated with Myozyme and switched to Lumizyme was included. This biochemical data demonstrated that alglucosidase alfa 4000L (Lumizyme) is comparable to alglucosidase alfa 160L (Myozyme).

For several years there have been two Genzyme products in the U.S. for the treatment of Pompe Disease - Myozyme (alglucosidase alfa, produced in 160L bioreactors) and Lumizyme (alglucosidase alfa, produced in 4000L bioreactors). The expanded Lumizyme indication has allowed Genzyme to review the current manufacturing processes for both products. Based on the expanded Lumizyme label, the sustainable production capabilities for Lumizyme, and the goal to align with current treatment practices worldwide, Genzyme will discontinue production of the Myozyme formulation and shift all production resources to Lumizyme.

Information for Patients Currently Treated with Myozyme

1. All **newly diagnosed Pompe patients** regardless of age and phenotype have access to Lumizyme effective immediately.

2. Myozyme will remain available to **existing patients** treated with Myozyme through December 31, 2014.

3. Patients currently receiving Myozyme: product will not be available for re-order after December 31, 2014. We recommend that you discuss the need to transition from Myozyme to Lumizyme with your doctor as soon as possible.

4. **Myozyme Clinical Study patients are not impacted at this time.**

We understand that members of the U.S. Pompe Community may have questions about their specific treatment plan. We encourage anyone with questions to reach out to their physician or health care facility directly for information. Patients with additional questions are encouraged to contact Genzyme Case Management at 1-800-745-4447, option 3. In addition, the US patient associations, the Acid Maltase Deficiency Association (AMDA) and the United Pompe Foundation (UPF) are available as resources.

We at Genzyme want to thank the U.S. Pompe Community for its continued support.

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INDICATION

Lumizyme® (alglucosidase alfa) is an enzyme replacement therapy for patients with Pompe disease (acid α-glucosidase (GAA) deficiency).

IMPORTANT SAFETY INFORMATION

If you or your child is taking Lumizyme, you should know that severe and potentially life-threatening allergic-type reactions known as anaphylaxis or severe hypersensitivity reactions, and other severe reactions related to the immune system may occur during and after Lumizyme treatment. Reactions may include, for example, kidney dysfunction, extreme difficulty breathing, shallow breathing, abnormal heart rate, low blood pressure, or throat tightness (including face, tongue and lip swelling) and skin lesions.

If you or your child has previously experienced these severe reactions, from any cause, you may require close observation during and after Lumizyme administration. You should discuss with your physician the signs and symptoms of anaphylaxis and hypersensitivity reactions. Anaphylaxis or severe hypersensitivity reactions are potentially very dangerous. If such a reaction is severe enough, your doctor may decide to immediately discontinue the infusion and provide you with immediate medical care. Appropriate medical support measures, including those that may be used to restart the heart or breathing, may be administered when you are being infused with Lumizyme. Should treatment be discontinued, your physician may discuss restarting therapy. You may require close observation during infusions with Lumizyme administration following a severe hypersensitivity reaction.

If you or your child has an illness (i.e. a chronic breathing condition) or has compromised heart function, there may be risk of acute heart and or lung failure, and your doctor may decide that close observation during and after Lumizyme administration may be necessary.

You or your child may be monitored for the development of systemic immune-mediated reactions while receiving Lumizyme. If these reactions occur, your doctor may discontinue the infusion and initiate appropriate medical treatment.

WARNING AND PRECAUTIONS

Anaphylaxis and Hypersensitivity Reactions: Life-threatening anaphylaxis and hypersensitivity reactions have been observed in some patients during and after treatment with alglucosidase alfa. If such a reaction is severe enough, your doctor may decide to immediately discontinue the infusion and provide you with immediate medical care.

Immune-Mediated Reactions: If you or your child has an illness (i.e. a chronic breathing condition) or has compromised heart function, there may be risk of acute heart and or lung failure, and your doctor may decide that close observation during and after Lumizyme administration may be necessary.

Risk of Acute Cardiorespiratory Failure: Infant Pompe patients with heart or breathing problems may be at risk for increasing the seriousness of these problems as a result of Lumizyme administration, and your child's doctor may require additional monitoring for these infants.

ADVERSE REACTIONS

The most frequently reported adverse reactions during Lumizyme studies in patients were allergy reactions and included: anaphylaxis, rash, fever, flushing/feeling hot, hives, headache, excessive sweating, nausea, cough, less oxygen in the blood, fast heart rate, rapid breathing, chest discomfort, dizziness, muscle twitching, agitation, bluish or purple skin, redness of skin, high blood pressure/increased blood pressure, facial paleness, chills, tremor, vomiting, fatigue, and muscle pain.

PLEASE SEE FULL PRESCRIBING INFORMATION

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SPECIAL POPULATIONS

If you are pregnant, you should use Lumizyme only if your doctor has determined that its use outweighs any risks to your unborn child.

To report suspected adverse reactions, contact Genzyme at 1-800-745-4447 or FDA at 1-800-FDA-1088 or **www.fda.gov/medwatch**.

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