



## United States Pompe Program Update, May 25, 2010

Genzyme would like to provide an update to the US Pompe community about the status of the Lumizyme™ (alglucosidase alfa) application in the United States. We are pleased to share that Lumizyme was approved by the US Food and Drug Administration (FDA) on May 24, 2010. The approved indication for Lumizyme is:

*Lumizyme (alglucosidase alfa) is a lysosomal glycogen-specific enzyme indicated for patients 8 years and older with late (non-infantile) onset Pompe disease (GAA deficiency) who do not have evidence of cardiac hypertrophy. The safety and efficacy of Lumizyme have not been evaluated in controlled clinical trials in infantile-onset patients, or in late (non-infantile) onset patients less than 8 years of age*

Please visit [www.lumizyme.com](http://www.lumizyme.com) for important safety information including boxed warning.

### Information about Lumizyme

Lumizyme is manufactured in Genzyme's Geel, Belgium facility at the 4000 liter (L) manufacturing scale and is filled into vials and labeled in the Waterford, Ireland facility.

Lumizyme requires the implementation of a restricted distribution program known as the **Lumizyme ACE (Alglucosidase Alfa Control and Education) Program**. The FDA approval of Lumizyme includes mandatory enrollment in this program for all prescribers, treatment sites, and patients to ensure that Lumizyme is administered to the intended patient population. Prior to administering Lumizyme, prescribers and sites where Lumizyme will be infused must complete training and enroll in the Lumizyme ACE Program. Prescribers also are responsible for enrolling their patients into the program.

Genzyme will be working closely with treating prescribers and infusion sites to facilitate enrollment into the Lumizyme ACE Program. For more information about the Lumizyme ACE program, please speak with your prescriber or contact a Genzyme case manager at 1-800-745-4447, option 3.

### Access to Treatment

With the approval of Lumizyme, there are now two approved enzyme replacement therapies for Pompe disease in the United States. Please keep in mind that Lumizyme and Myozyme® (alglucosidase alfa) are two different products.

In order to preserve the supply of Myozyme for infants and children who are restricted from using Lumizyme, it is necessary for all patients who are eligible for treatment with Lumizyme to receive therapy with this product. Please review the following information to better understand which product is indicated for specific patient populations in the US.

- ***Patients under the age of 8 with either infantile-onset or late-onset Pompe disease***

All patients under the age of 8 should continue to receive treatment with Myozyme. Patients in this category who are diagnosed with late-onset Pompe disease **who do not have evidence of cardiac hypertrophy** will transition to Lumizyme when they reach 8 years of age. Patients diagnosed with infantile-onset Pompe disease will remain on Myozyme regardless of age.

- ***Patients of any age with a confirmed diagnosis of infantile-onset Pompe disease or evidence of cardiac hypertrophy (enlarged heart)***

Patients with infantile-onset Pompe disease will receive treatment with Myozyme, regardless of age. If you are an infantile-onset patient currently receiving Myozyme, you will continue to receive treatment without any changes. Patients with evidence of cardiac hypertrophy also should receive treatment with Myozyme, regardless of age. Your treating physician can confirm if you have cardiac hypertrophy and should speak with a Genzyme Medical Director regarding treatment options. Please contact a case manager, local Genzyme representative, or your physician with questions.

- ***Patients 8 years of age and older with late-onset Pompe disease and currently enrolled in clinical studies (e.g. ATAP, E-IND, Dosing study)***

Patients currently enrolled in or receiving treatment via ATAP have **until August 20, 2010** to transition to commercial Lumizyme. The ATAP program will be discontinued as of August 20<sup>th</sup>. If you are currently enrolled in an E-IND or the Dosing Study, please contact your prescribing physician for more information about transitioning to commercial treatment. Patients in these three clinical studies should plan to transition to commercial Lumizyme **unless they have evidence of cardiac hypertrophy or a diagnosis of infantile-onset Pompe disease**. If you meet either of these conditions, please speak with your treating physician to determine if Myozyme is appropriate for you instead of Lumizyme. Genzyme case managers and field staff will be working with treating prescribers and infusion sites to aid with the transition process and enrollment into the Lumizyme ACE Program. Enrollment in the program is required before Lumizyme can be shipped. Please contact a case manager, local Genzyme representative, or your physician with questions.

- ***Patients 8 years or older with late-onset Pompe disease currently receiving Myozyme who do not have evidence of cardiac hypertrophy***

In order to preserve the supply of Myozyme for infants and children, it is necessary for all patients who are eligible for treatment with Lumizyme to receive therapy with this product. You should plan to transition to Lumizyme **by August 20, 2010** to minimize the risk for missed infusions. Genzyme case managers and field staff will be working with prescribers and infusion sites to aid with the transition process and enrollment into the Lumizyme ACE Program. Enrollment in the program is required before Lumizyme can be shipped. Please contact a case manager, local Genzyme representative, or your physician with questions.

- ***Patients 8 years and older with late-onset Pompe disease who are interested in initiating treatment and do not have evidence of cardiac hypertrophy***

Patients who have not yet been on treatment who are 8 years and older with late-onset Pompe disease and no evidence of cardiac hypertrophy may receive treatment with Lumizyme. Please contact a case manager in Genzyme Care Coordination for information about obtaining access to therapy. Genzyme will work with your prescriber and infusion sites to complete enrollment in the Lumizyme ACE Program. Enrollment in the program is required before Lumizyme can be shipped.

We will continue to keep you informed of relevant updates in the coming months. In the meantime, we encourage patients to contact their physician, Genzyme case manager at 1-800-745-4447 option 3, Genzyme Medical Information at 1-800-745-4447 option 2, or local Genzyme representative with any questions you may have. We are pleased to be able to now offer broader access to treatment for the full US Pompe patient community and greatly appreciate your ongoing support during this process.