POMPE Connections

Indication, effectiveness and safety of Myozyme

Myozyme is now on the market for Pompe patients in many countries around the world. For many people, just like you, the mere fact that there is a treatment to fight the symptoms of Pompe disease is welcome news. Of course, before you can begin treatment with Myozyme which will likely be a lifelong treatment, you need to know whether you are a potential candidate and if the drug will be reimbursed. If you have been diagnosed with Pompe disease, then the answer is likely, yes. Naturally, you are concerned about how effective and safe the treatment will be for you, your child or your family member. Genzyme has conducted a number of clinical studies to evaluate the safety and efficacy of Myozyme, and has ongoing studies to continue researching the treatment. This handout provides you with the information to help you make informed decisions about your potential Myozyme treatment.

Q Who should take Myozyme?

A Myozyme is an enzyme replacement therapy (ERT) for Pompe disease. In Europe the label for Myozyme reads: *Myozyme is indicated for long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid alfa glucosidase deficiency). The benefits of Myozyme in patients with late-onset Pompe disease have not been established.*

In the United States, the label for Myozyme reads: *Myozyme (alglucosidase alfa) is indicated for use in patients with Pompe disease (GAA deficiency). Myozyme has been shown to improve ventilator-free survival in patients with infantile-onset Pompe disease as compared to an untreated historical control, whereas use of Myozyme in patients with other forms of Pompe disease has not been adequately studied to assure safety and efficacy.*

The label in Canada is similar to that of the United States.

Q What are the results of clinical trials of Myozyme?

A A number of clinical trials have been conducted for both infant and older Pompe disease patients. The results from many of these studies led to the approval of Myozyme

Supported by an unrestricted grant from genzyme

Other names for Pompe disease
Acid alpha-glucosidase deficiency, acid maltase deficiency (AMD), glycogen storage disorder (GSD) type II, glycogenosis II, and lysosomal alpha-glucosidase deficiency. In different parts of the world, Pompe may be pronounced *"pom-PAY," "POM-puh,"* or *"pom-PEE."*

in the European Union, United States and Canada, and will be used to help support approval of Myozyme in many additional countries over the next several years.

A pivotal clinical study, initiated in 2003, has been of particular note in demonstrating Myozyme's efficacy and safety. In this study, 83 percent of patients treated with Myozyme were both alive and free of invasive ventilator support at 18 months of age, compared with two percent of patients in the historical group. The trial enrolled 18 infants with Pompe disease. These patients began receiving therapy at six months of age or less. Approximately 39 percent of patients treated with Myozyme developed infusion associated reactions, which were mostly mild to moderate. Two patients experienced serious infusion reactions.

In a second clinical trial, 21 older infants and toddlers with rapidly progressive and more advanced Pompe disease (age 6 months to 3.5 years) received 20 mg/kg Myozyme once every 2 weeks for 52 weeks (26 infusions). After 52 weeks of treatment results showed a survival rate of 73 percent compared to the corresponding survival figure of 37 percent in an untreated reference group. In 10 patients who were free of invasive-ventilator support when the study began, 50 percent remained so after 52 weeks of treatment.

For more information on Myozyme clinical trials, please visit www.clinicaltrials.gov or www.worldpompe.org.

Q **How effective is Myozyme?**

A The vast majority of infants with Pompe disease, who were treated with Myozyme, showed improvement in cardiac function as well as stabilization or improvements in growth parameters.

Motor and respiratory responses to treatment have been more variable. Infants with Pompe disease, who demonstrated motor gains, had greater preservation of motor function and lower glycogen content in the quadriceps muscle at the initial point of clinical trials. A higher proportion of patients with better motor outcomes show stability or improvement in growth parameters (especially weight). The majority of infant patients, regardless of motor outcomes or features measured at the beginning of clinical trials, show reversal of cardiomyopathy (a disorder of the heart muscle).

Data results indicate that the earlier the diagnosis and treatment for infant patients, the greater the potential for positive outcomes.

Q **How safe is Myozyme? (See also Treatment Precautions section)**

A No formal studies have been conducted to determine how Myozyme may interact with other medicines. However, since Myozyme is a recombinant human protein, it is unlikely that there would be drug-to-drug interactions.

No studies on the ability to drive and use machines have been conducted with Myozyme. No testing has been performed on the interaction between alcohol and Myozyme.

Myozyme should not be used during pregnancy unless absolutely necessary. It is recommended to stop breast-feeding during treatment with Myozyme as alglucosidase alfa may be excreted in breast milk.

Appropriate medical support measures should be readily available when Myozyme is administered. For detailed information on the potential side effects of Myozyme, visit the following websites:

If you live in the United States or other countries outside of Europe, visit www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm?fuseaction=Search.Label_ApprovalHistory.

If you live in Europe, visit www.emea.eu.int/humandocs/Humans/EPAR/myozyme/myozyme.htm.