POMPE Connections

Treatment precautions for Myozyme

There is some degree of risk associated with any medical treatment. Understanding the risks, and being prepared to take precautions in order to minimize these risks is vital to managing treatment with Myozyme. This handout explains the type of risks that you may be exposed to, and how physicians can safely administer Myozyme to minimize these risks.

Q **What are some of the risks associated with Myozyme?**

A A number of clinical studies have been undertaken using Myozyme with Pompe disease patients. Approximately 39% of patients treated with Myozyme in these studies have developed infusion adverse reactions (IARs). Most of the reactions were mild to moderate but some severe reactions have also been reported. During these studies, the most common side effects (more than 1 patient in 10) occurred during or after the infusion. These side effects included flushing, urticaria (an itchy rash), pyrexia (fever) and rash. That's why you will be closely monitored during and after infusions.

If you have advanced Pompe disease, then you may have compromised cardiac and respiratory function. This may predispose you to a higher risk of severe complications from infusion-associated reactions. You should be closely monitored during administration of Myozyme.

An acute illness (for example, a cold, pneumonia or infection) at the time of your Myozyme infusion may also increase your risk of developing adverse reactions. Careful consideration will be given to your clinical status prior to administration of Myozyme.

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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."*

You may also develop antibodies (proteins that are produced in response to Myozyme). In two clinical trials, the majority (89%) of patients tested positive for IgG antibodies to alglucosidase alfa. Most patients who develop antibodies do so within the first three months of exposure. Some patients who develop antibodies may have a poorer clinical response to treatment, or may lose motor function as levels of antibodies rise. These patients should be monitored closely.

If you are hypersensitive (allergic) to alglucosidase alfa or any of the other ingredients in Myozyme, then you should not receive this treatment.

You should report any potential side effects you may be experiencing or questions you might have about the safety of Myozyme to the treating team.

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.

Q **What are the recommended precautions associated with Myozyme?**

A Approximately 39% of patients treated with Myozyme have developed infusion adverse reaction (IARs). Some reactions were severe. Appropriate medical support measures should be readily available when Myozyme is administered.

If you experience reactions when receiving Myozyme infusions, then you will be treated with caution on subsequent infusions. Mild and transient effects may not require medical treatment or discontinuation of the infusion. Your reactions can be managed by reducing the infusion rate, temporary interruption of the infusion or pre-treatment with an oral antihistamine and/or antipyretics. You can have a reaction at anytime during the infusion of Myozyme, or generally up to two hours after the infusion. Reactions are more likely with higher infusion rates.

If you are pregnant, Myozyme treatments should not be used unless clearly necessary. It is recommended to stop breast-feeding when Myozyme is used as alglucosidase alfa may be excreted in breast milk.

Q **Where is there more information on the safe use of Myozyme?**

A If you have questions regarding the safe use of Myozyme, please speak directly with your physician.