

## Preparation, administration and monitoring of Myozyme

**T**he first time that you receive Myozyme will probably be a totally new experience for you. You'll likely have plenty of questions – and rightly so. After all, you've never been through something like this before. You may be wondering: What should I expect? What does Myozyme look like, and how do I get it into my body? What if I hate needles? How long will it take? What do I do during the treatment? Consider this handout your first step towards getting some answers on how Myozyme is prepared and administered, what the treatment will be like, and how your body may react. Be sure to jot down any other questions that you may have and discuss them with your physician before your treatment.



### **Q** How is Myozyme prepared?

**A** Myozyme comes in a glass vial and is kept in the refrigerator prior to use. It looks like a white or off-white powder. A pharmacist will reconstitute (mix) Myozyme one vial at a time with sterile water prior to administering it to a patient. After it is mixed, it appears as a clear, colorless to pale yellow solution (occasionally some particles may be present in the mixed solution but will not impact the efficacy of the treatment). To prevent protein particles from entering your body, it's necessary to have a filter placed between your infusion line and your body. Once the Myozyme has been mixed, it is only stable and able to be administered to the patient for a short period of time. The mixing process can take several hours depending on the number of vials being used.

### **Q** How is Myozyme administered?

**A** After Myozyme has been mixed with water, it is placed in a special infusion bag which will then be injected intravenously (through the bloodstream). Before administering Myozyme your nurse or physician will explain the procedure to you. During the infusion, you will be carefully monitored to observe any responses that you may have to the treatment and provide any required interventions. It's possible that during the infusion a protein particle will block the filter. When this happens, the infusion pump will sound an alarm and the filter will need to be exchanged.

At the end of each treatment session, it is important that the infusion line be 'flushed' with saline (solution of sodium chloride in

#### **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."

sterile water). This ensures that all of the Myozyme is properly flushed from the equipment and administered.

The amount of Myozyme that you receive is dependent on your body weight. The recommended dosage is 20 mg/kg body weight administered every 2 weeks. In some clinical studies, Pompe patients received doses up to 40 mg/kg body weight.

The normal procedure is that the infusion line is inserted in one of your veins. This can be done easily if you have good veins that can be easily accessed. However, some patients do have small veins or veins that can't be accessed easily. In particular, infants and children tend to have very small veins. For these patients, a port-a-cath or PICC line may be an option.

A port-a-cath is implanted under your skin. This is a small device that lets you receive infusions without a needle. The advantage is that there is easy access to your veins. The disadvantage of this type of device is that there is a higher risk of infection than there would be with a typical intravenous infusion.

A PICC line is a long, thin, flexible tube known as a catheter. It is inserted into one of the large veins of the arm near the bend of the elbow. It is then pushed into the vein until the tip sits in a large vein just above the heart. The PICC line can be used to give you Myozyme treatments. It can also be used to take blood samples. The PICC can be left in for weeks or months. This lets you receive treatment without the need for frequent needles. The disadvantage of a PICC line is again that there is a higher risk of infections.

If your veins are difficult to access it is a good idea to talk about these options with your physician.

**Q**

**What if I do have good veins but am afraid of needles?**

**A**

Everyone has a different reaction to needles. You are not the only one who may be uncomfortable about the thought of frequent injections. The important thing to remember is that healthcare specialists are there to help you. Talk to them about your concerns before you start your infusion. You can read more about how to manage your fear of needles by downloading the brochure titled – ‘*Needle Fear*’ from the International Pompe Association website at [www.worldpompe.org/NeedleFear.pdf](http://www.worldpompe.org/NeedleFear.pdf).

**Q**

**What should I expect during a Myozyme infusion?**

**A**

Your Myozyme infusion session will typically last about 4 to 5 hours. When you first arrive for your treatment session, your healthcare specialist will explain the procedure to you and record your relevant medical information. You may have to wait for a short while before your infusion begins. That's because Myozyme must be used soon after it is mixed. When it is ready, you will be hooked up to the intravenous tubing which in turn is connected to a special electronic pump which infuses Myozyme into your body.

During the treatment session you can read, watch television, exercise, or simply lie back and relax. Children are able to play games, read or watch a video. If necessary, you can visit the bathroom. Your healthcare specialist will monitor you as the infusion progresses. Be sure to communicate how you are feeling, or if you have any concerns.

**Q**

**What if I have a reaction during an infusion?**

**A**

The majority of side effects during a Myozyme infusion have been mild to moderate. These reactions have included such things as nausea, vomiting, headache, cough, and agitation. If you have a mild or transient reaction, you may be able to still continue with the infusion. In the case of a more severe reaction, the infusion rate may be slowed down or immediately halted.

Some patients have received an oral antihistamine or an antipyretic prior to an infusion treatment. This approach has helped to manage minor reactions.

If you feel sick or have a fever before your treatment, it's important to tell your physician or nurse. Side effects may appear earlier when you are not feeling well.

Of course, with any medical treatment, there is always the risk of an allergic reaction. If you have a severe adverse reaction, then your healthcare specialist will immediately stop the infusion and provide the appropriate medical care.

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\\_Approval\\_History](http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm?fuseaction=Search.Label_Approval_History).

If you live in Europe, visit [www.emea.eu.int/humandocs/Humans/EPAR/myozyme/myozyme.htm](http://www.emea.eu.int/humandocs/Humans/EPAR/myozyme/myozyme.htm).

**Q**

**How should I expect to feel in the days following an infusion?**

**A**

You may feel tired right after your infusion and during that evening. After all, spending a whole day in the hospital can make you feel sluggish. You'll regain your energy and feel much better after a few days. Some patients remain active and energized the whole period between infusions. Others feel a decline of energy just before the next infusion. Everyone responds on an individual basis before and after infusions.

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