

## Outcomes of Myozyme treatment

**R** eceiving enzyme replacement therapy with Myozyme will no doubt be a life-altering experience – not only from a physical perspective but also from a mental and emotional perspective. In the short-term, you will be busy with the details of arranging your treatment appointments and coping with any pre and post-treatment issues that arise. But it's also critical to devote some time to think about how you will deal with the longer-term effects of treatment. What if it takes longer than you expect to observe the benefits of treatment? How will you manage the possible roller-coaster ride of emotions that you may feel? And how should you plan for your future, considering that Myozyme treatment will likely prolong your life? This handout provides you with information on what to expect, and how to best prepare yourself and your family.



### **Q** What should my family, friends and I expect from my treatments?

**A** You may not experience the benefits of Myozyme right away; however, Myozyme is still working in your body as it makes its way through your system. You may need to have multiple infusions over a period of several months before you may begin to notice any difference. That's because Myozyme is an enzyme replacement therapy. It replaces the enzyme alpha-glucosidase which is deficient or missing from your body. Before the alpha-glucosidase in Myozyme

starts to exhibit its positive effects, it has some work to do. It must clear some of the excess glycogen that is stored in your muscles and tissues. This takes time. The amount of time depends on the severity of your condition. The less severe your Pompe condition, the earlier you may observe improvement. But even some severely affected Pompe patients, who started Myozyme in the expanded access program, have experienced improvement. Some of these patients report that they don't experience further deterioration. Some patients also claim to have more energy,

#### **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."

## Outcomes of Myozyme treatment (continued)

---

better breathing, increased appetite and easier ability to swallow. Visit the International Pompe Association website, [www.worldpompe.org/testimonials.html](http://www.worldpompe.org/testimonials.html) to read patient stories about living with Pompe and their experiences with Myozyme. It is important to remember that every individual reacts differently to the enzyme. There is no guarantee that Myozyme will have the same effect on each person.

### **Q** How can I best manage my expectations of Myozyme treatment?

**A** The benefits of Myozyme treatment will not happen overnight. It may take months to see any effects, and some patients may not experience visible results. So while you may be very hopeful when you start treatment, this hope may fade as time passes. It may be difficult to keep your spirits up, and to continue with the physical exercises that you are given. You may also experience feelings of uncertainty and impatience. These are all normal responses.

The challenge is to maintain a positive mental outlook despite the inevitable ups and downs. After all, getting Myozyme treatment represents a huge step and the potential for a new future. To finally have treatment available for Pompe disease is an unbelievable and unparalleled experience. So keep your spirits up and think about all the good things that can result from treatment – a chance to improve your breathing and your mobility, a better quality of life, and more time to spend with family and friends.

When you start to experience improvement in your physical condition, stay true to your regimen of exercises and healthy diet. This approach will provide you with the fullest benefit from your Myozyme treatment. You might even consider trying new exercises – such as breathing exercises – that you weren't able to do before treatment. Of course, be sure that all of your exercises are supervised by an expert like a physiotherapist or a speech therapist. If you do not experience improvement you should also discuss this with your specialist.

### **Q** How should I plan for my future?

**A** In the past, if you were diagnosed with Pompe disease, you faced a future filled with physical and emotional challenges. If you can receive treatment with Myozyme, hopefully further deterioration will be reduced and you'll likely experience physical improvements.

The availability of treatment requires a shift in your mindset. Although no one knows for certain how much you will benefit from Myozyme treatment, you can certainly consider a new perspective on the future. You'll likely live longer due to your treatment. So although Myozyme needs time to show its effects, you can use that time to adjust your thinking to a more long-term outlook. You may want to think about suitable housing, your job and career growth, old age pension, long term relationships and rehabilitation services. Coping with Pompe disease will become an issue of how you manage a chronic condition in the most positive way possible.

---

This publication is designed to provide general information in regard to the subject matter covered. It is distributed as a public service by the International Pompe Association, with the understanding that the International Pompe Association is not engaged in rendering medical or other professional services. Medicine is a constantly changing science. Human error and changes in practice make it impossible to certify the precise accuracy of such complex materials. Confirmation of this information from other sources, especially one's physician, is required. Please keep in mind that the effectiveness of Myozyme varies from person to person.