

Practical considerations and supportive management for treatment with Myozyme

You've gone through the process of finding a physician and completing all the necessary tests to determine your eligibility to receive Myozyme treatment. Reimbursement issues are being resolved. Now it's time to think about some practical issues related to your upcoming treatment. How do you plan to get to and from the hospital? And how do you intend on keeping up with the demands of your job, family care or school studies? As your treatment continues over time, you may have the choice of receiving treatment at home. So the details of that decision also need to be mapped out. And finally, you need to remember that what you eat and how you exercise your body during treatment will also contribute to your ultimate well-being. This handout touches on all these points to help you succeed in the planning and management of your Myozyme treatment.



Q What practical arrangements should I make to accommodate my treatment?

A Once your Myozyme treatment has been arranged, you need to set aside some time to think about some very practical things. Each treatment will take about 4 to 5 hours. Realistically, you should allow about one full day for each infusion session. You'll need to give yourself time to get to the hospital, wait for a short time before your infusion appointment, have the infusion, and then if necessary, wait again after the infusion to make sure that there are no adverse effects. Then of course, you need to factor in the time to return home.

You may find that you are tired after your day at the hospital, so think about whether you should drive to the hospital or arrange for a ride there and back. It may be a smart idea to have someone accompany you to the hospital to provide support before, during and after the treatment.

Q How will treatment affect my work or school studies?

A Your Myozyme treatment will likely require you to spend one day every two weeks at the hospital. You will need to arrange for that time off from your job or school or additional care for your children at home. Talk to your employer or school director and explain your

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."

situation. Some countries may have special regulations in place which make it easier to receive time off for medical treatment. If you are a student, ask your teacher to provide you with the work that you'll miss so you can keep pace with your studies.

Q Am I able to skip a treatment when necessary? For example when I go on holiday?

A Myozyme is a life-long treatment and it is important that you follow the schedule. However, in most situations it will be possible to negotiate with your physician to skip a treatment to be able to go on holiday or when you don't feel well. In the case of a holiday it's important to discuss a change in your infusion schedule well in advance with your physician. Your physician is responsible for informing the pharmacist and the medical staff of your absence. A skipped infusion can be received before or directly after your holiday or made up in other ways. It's important to look at the amount of days between your infusions and available options should be discussed with your physician. For special events like weddings or funerals that take place on your infusion day it's also possible to ask your physician to get the infusion on another day, for example a day earlier or later. In this case it's also important to discuss this with your physician as early as possible.

Q Can I receive treatment at home?

A At the beginning of your Myozyme treatment, you will be treated in the hospital. Myozyme is not an everyday medication, and its administration needs to be carefully

monitored by medical specialists. However, as your treatment progresses – and if the physician agrees that it is a good idea and the law and reimbursement plan in your country allows it – you may be able to receive infusions from the comfort of your home. Of course, administration in a home environment must be carefully organized. The Myozyme infusion may need to be prepared by the hospital pharmacist, then either picked up at the hospital or delivered to your home. A trained professional home-care nurse would then go to your home and administer the treatment. In that situation, you should also arrange to have a family member or a friend with you during and after the infusion. This person should know what to do or who to call in the case of any treatment difficulties.

Q How should I manage my diet and exercise during treatment?

A For everyone – and especially if you have Pompe disease – it is important to pay attention to your nutritional and physical habits. Arrange to meet with a dietician or a nutritionist for advice on the type of food or additional vitamins and minerals that you should eat when you are receiving treatment.

Make an appointment for an overall physical examination before you start treatment. Osteoporosis is a common problem in people with mobility impairment, so that condition should be closely monitored.

Physical exercise is also a very important component of your overall wellness plan. Be sure to talk to your physician or to a physical therapist about the type of exercise that will help you improve your body and optimize your physical abilities. For more

information, read the publication *'Exercise and Physical Therapy'* in the Pompe Connections brochure available on the website www.worldpompe.org/pompeconnections.html.

Q **Will I be able to get reimbursement for my treatment and, for example, travel expenses to the hospital?**

A Since every country has its own health care system and regulations we recommend you discuss reimbursement with your physician or patient organization. When needed, you can also contact the Genzyme office in your country. You can find the phone numbers and addresses of Genzyme's worldwide offices at www.genzyme.com/global_locations/us/us_global_locations.asp.

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.