

## The cost of Myozyme

**I**t has taken many years of dedicated research and development by medical experts and scientists to produce Myozyme as a treatment for Pompe disease. In fact, Myozyme is the world's first, and currently the only disease-specific treatment for this genetic disease. This handout explains the details associated with the cost of Myozyme and the rationale behind this cost. It also discusses the types of options that may be available to help you afford receiving this treatment.



### **Q** How much does Myozyme cost?

**A** Enzyme replacement therapies (ERT), including Myozyme, are not inexpensive drugs. In the United States, and throughout the rest of the world, the price for Myozyme will be relatively similar. An individual's treatment cost will vary based on a patient's weight, since the Myozyme is dosed per kilogram of body weight. Your physician can provide you with further details on the specific cost of being treated with Myozyme.

### **Q** How is the cost determined?

**A** The price of all of Genzyme's enzyme replacement therapies are determined by three factors – the cost of research and product development, the cost of manufacturing of a complex protein by recombinant DNA technology, and the rarity of the conditions being treated.

Genzyme has invested more than \$500 million as of 2005 to develop and manufacture a therapy for Pompe disease, and anticipates investing an additional several hundred million dollars in the coming years to support ongoing clinical

#### **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."

trials, continued research, and post approval commitments required by regulatory authorities. Since Pompe disease is so rare, these costs are spread over a very small group of patients. As a result, the per-patient cost of treatment is higher than for typical drugs treating more common disorders.

Genzyme also re-invests funds into further research and development programs for next generation Pompe therapies and to address other new potential therapies for unmet medical needs.

### **Q Who pays for my treatment with Myozyme?**

**A** Like other enzyme replacement therapies, reimbursement for Myozyme varies depending on the country in which you live and your health care benefits. In the United States, reimbursement for treatment depends on your type of insurance plan. In Europe and other regions, reimbursement is typically managed nationally based on each country's health system.

If you live in the United States, you can contact Genzyme Treatment Support at 800-745-4447, option 3, to help better determine your insurance coverage or help explore alternative coverage options for treatment. If you live outside of the United States, contact your treating physician or national patient association for more information.

**Q**

### **Are there charitable programs that can help me to afford Myozyme treatment?**

**A**

Genzyme manages a Charitable Access Program for qualified patients who do not have insurance coverage in the United States, and has other programs in place to help with access internationally.

National health authorities in some countries may not currently support treatment with Myozyme. In those cases, Genzyme's International Charitable Access Program provides temporary access to treatment for patients in a serious or life-threatening medical condition until reimbursement in that country is obtained.

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