



Genzyme Corporation  
500 Kendall Street  
Cambridge, MA 02142  
www.genzyme.com

**For Immediate Release**  
April 28, 2006

Media Contact:  
Bo Piela  
(617) 768-6579

Investor Contact:  
Kristen Galfetti  
(617) 768-6563

## **FDA Approves Genzyme's Myozyme® for All Patients with Pompe Disease**

CAMBRIDGE, Mass.—Genzyme Corp. (Nasdaq: GENZ) announced today that the Food and Drug Administration has granted marketing approval for Myozyme® (alglucosidase alfa) in the United States. Myozyme has been approved for the treatment of patients with Pompe disease, a debilitating, progressive and often fatal disorder affecting fewer than 10,000 people worldwide. The product is the first treatment ever approved for Pompe disease and the first for an inherited muscle disorder.

“This is a special day for people across the Pompe community and at Genzyme, who have worked together for many years and overcome enormous challenges so that patients with this devastating disease now have a chance,” said Henri A. Termeer, chairman and chief executive officer of Genzyme Corp.

The Myozyme label includes the following indication: “Myozyme (alglucosidase alfa) is indicated for use in patients with Pompe disease (GAA deficiency). Myozyme has been shown to improve ventilator-free survival in patients with infantile-onset Pompe disease as compared to an untreated historical control, whereas use of



Myozyme in patients with other forms of Pompe disease has not been adequately studied to assure safety and efficacy.”

The product label also includes a boxed warning with information on the potential risk of hypersensitivity reactions associated with Myozyme. The boxed warning states that “Life-threatening anaphylactic reactions, including anaphylactic shock, have been observed in patients during Myozyme infusion. Because of the potential for severe infusion reactions, appropriate medical support measures should be available when Myozyme is administered.” Of the 280 patients who received Myozyme in clinical studies or through expanded access, eight patients (3 percent) experienced severe or significant hypersensitivity reactions. Full prescribing information for the product is available on Genzyme’s Web site:

[http://www.genzyme.com/components/highlights/mz\\_pi.pdf](http://www.genzyme.com/components/highlights/mz_pi.pdf)

Pompe disease manifests as a broad spectrum of clinical symptoms. All patients typically experience progressive muscle weakness and breathing difficulty, but the rate of disease progression can vary widely depending on the age of onset and the extent of organ involvement. When symptoms appear within a few months of birth, babies frequently display a markedly enlarged heart and die within the first year of life. When symptoms appear during childhood, adolescence or adulthood, patients may experience steadily progressive debilitation and premature mortality due to respiratory failure. They often require mechanical ventilation to assist with breathing and wheelchairs to assist with mobility.

Genzyme recently completed enrollment in its clinical trial involving patients with late-onset Pompe disease. Ninety patients have been enrolled in this international,



placebo-controlled study. Currently, more than 280 patients in 30 countries are receiving Myozyme through clinical trials, expanded access programs, or pre-approval regulatory mechanisms.

Myozyme has received orphan drug designation in the United States, which provides seven years of market exclusivity. The orphan drug law is designed to encourage the development of treatments for rare disorders such as Pompe disease, for which no therapies have existed previously. Genzyme expects to launch Myozyme in the United States within two weeks. Late last month, Myozyme was approved in the European Union.

Because early diagnosis, intervention and treatment are critical in Pompe disease and other lysosomal storage disorders, Genzyme has for the past seven years supported several outside research collaborations to develop new diagnostic technology. This research has led to the recent introduction of an enzyme assay utilizing blood samples that makes it possible to diagnose Pompe patients more rapidly and with a less-invasive procedure. Genzyme will offer this test now through its Genzyme Genetics unit, and the test will also be available through several other clinical laboratories in the United States and elsewhere in the world.

“The journey from development to approval of a therapy for Pompe disease has been a long and winding road, but we are now at a milestone and are thrilled with the outcome,” said Randall H. House, chairman of the International Pompe Association and president of the Acid Maltase Deficiency Association (AMDA), a Pompe patient association in the United States. “Enzyme replacement therapy with Myozyme gives



Pompe patients hope." The AMDA, formed in 1995, has assisted in funding Pompe disease research and promotes public awareness of Pompe disease.

Valerie Cwik, medical director for the Muscular Dystrophy Association, said: "Myozyme is the first treatment for any of the muscle diseases included among the 40 neuromuscular disorders covered by the Muscular Dystrophy Association. This is a great day for people with Pompe disease, and a hopeful moment for the thousands of other people who are affected by the diseases in the MDA program, because it shows that support and research can lead to treatments." The MDA helped support patients who took part in clinical trials of Myozyme and also sponsored early research in Pompe disease.

Genzyme began working to develop a treatment for Pompe disease in 1998. In 2003, the company initiated a pivotal clinical study of Myozyme that demonstrated the product's safety and efficacy. In the study, 83 percent of patients treated with Myozyme were both alive and free of invasive ventilator support at 18 months of age. In a natural history study, 2 percent of similar infantile-onset patients were alive at 18 months of age. The pivotal trial enrolled 18 patients with infantile-onset Pompe disease, who began receiving therapy at approximately six months of age. The most common serious adverse events observed in clinical studies of Myozyme, whether or not they were related to the drug, were pneumonia, respiratory failure, respiratory distress, catheter-related infection, respiratory syncytial virus infection, gastroenteritis and fever. Many of these can be complications of Pompe disease.

"We are very proud that we have been able to bring to market four therapies for ultra-orphan diseases where no treatments existed previously," said Mr. Termeer. "This



underscores our fundamental commitment to patients and confirms the productivity of our research efforts. We continue to invest in potential new approaches to treating these diseases.”

Myozyme is the fourth enzyme replacement therapy developed by Genzyme for a rare genetic disease. Genzyme has developed Cerezyme® (imiglucerase for injection) for Type 1 Gaucher disease; Fabrazyme® (agalsidase beta) for Fabry disease; and, in collaboration with BioMarin Pharmaceutical Inc., Aldurazyme® (laronidase) for MPS I. These treatments are currently available to patients throughout the world.

Genzyme currently manufactures Myozyme in the United States. In the future, the company expects to also produce Myozyme at its new protein manufacturing facility in Geel, Belgium, and its new fill/finish facility in Waterford, Ireland, to ensure that it is able to meet the anticipated demand for the product throughout the world.

### About Pompe Disease

Pompe disease, also known as Acid Maltase Deficiency or Glycogen Storage Disease Type II, is one of more than 40 genetic diseases called lysosomal storage disorders, which are caused by a deficiency or malfunction of specific enzymes found in cell lysosomes. People born with Pompe disease have an inherited deficiency of an enzyme known as acid alpha-glucosidase (GAA). Enzymes, which are protein molecules within cells, trigger biochemical reactions in the body. In a healthy person with normal GAA activity, this particular enzyme would assist in the breakdown of glycogen, a complex sugar molecule stored within a compartment of the cell known as the lysosome. But in Pompe disease, the GAA activity may be dramatically reduced, dysfunctional, or non-existent, resulting in an excessive accumulation of glycogen in the lysosome.

Eventually, the lysosome may become so clogged with glycogen that normal cellular function is disrupted and muscle function is impaired. Although there is glycogen storage in the cells of multiple tissues, heart and skeletal muscles are usually the most seriously affected.

For more information on Pompe disease, please visit [www.pompe.com](http://www.pompe.com).



For information on Myozyme ordering and support services please call (800) 745-4447 or (617) 768-9000.

### About Genzyme

One of the world's leading biotechnology companies, Genzyme is dedicated to making a major positive impact on the lives of people with serious diseases. This year marks the 25<sup>th</sup> anniversary of Genzyme's founding. Since 1981, the company has grown from a small start-up to a diversified enterprise with more than 8,000 employees in locations spanning the globe and 2005 revenues of \$2.7 billion. Genzyme has been selected by FORTUNE as one of the "100 Best Companies to Work for" in the United States.

With many established products and services helping patients in more than 80 countries, Genzyme is a leader in the effort to develop and apply the most advanced technologies in the life sciences. The company's products and services are focused on rare inherited disorders, kidney disease, orthopaedics, cancer, transplant and immune diseases, and diagnostic testing. Genzyme's commitment to innovation continues today with a substantial development program focused on these fields, as well as heart disease and other areas of unmet medical need.

This press release contains forward-looking statements regarding the anticipated timing of a Myozyme launch, plans to launch a diagnostic test for Pompe disease, and the expected addition of Myozyme manufacturing sites. These statements are subject to risks and uncertainties that could cause actual results to differ materially from those projected, including our ability to successfully identify and market to new patients; the timely receipt of pricing and reimbursement approvals in approved countries; our ability to enter into agreements with our distributors; scientific, technical and manufacturing issues that could prevent the successful launch of the Pompe diagnostic test, the failure of the test to produce diagnostic results as anticipated; the commercial acceptance of the diagnostic test, including the acceptance of the diagnostic test at price levels that are economically viable for Genzyme Genetics; and our ability to produce Myozyme at our Geel, Belgium facility or produce it in sufficient quantities to meet demand. Please refer to the risks and uncertainties described in reports filed by Genzyme with the Securities and Exchange Commission under the heading "Factors Affecting Future Operating Results" in the Management's Discussion and Analysis of Financial Condition and Results of Operations section of Genzyme's Annual Report on Form 10-K for the year ended December 31, 2005 for a more complete discussion of the risks associated with Genzyme's business. Genzyme cautions investors not to place substantial reliance on the forward-looking statements contained in this press release. These statements speak only as of the date of this press release, and Genzyme undertakes no obligation to update or revise the statements.



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### **Conference Call Information**

Genzyme will host a conference call today at 2:00 p.m. Eastern time to discuss FDA approval of Myozyme. To participate in the call, please dial (706) 679-8722 and refer to conference ID number 8684852. A replay of this call will be available until midnight on May 5. To listen to the replay, please dial (706) 645-9291.