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Media Contact:
Lori Gorski
(617) 768-9344
lori.gorski@genzyme.com

Investor Contact:
Emily Merchant
(617) 768-6140

Genzyme Receives FDA Approval for Lumizyme for Pompe Disease

CAMBRIDGE, Mass. – Genzyme Corporation (Nasdaq: GENZ) announced today that the FDA has granted U.S. marketing approval for Lumizyme™ (alglucosidase alfa), produced at the 4000 liter (L) bioreactor scale at its manufacturing facility in Geel, Belgium. Lumizyme is the first treatment approved in the United States specifically to treat patients with late-onset Pompe disease.

“This is an important day for the Pompe community, especially for those patients with late-onset Pompe disease in the United States who are awaiting treatment for this devastating disease,” said Genzyme Chairman and Chief Executive Officer, Henri A. Termeer. “We are grateful to the FDA for their efforts to approve Lumizyme ahead of its scheduled PDUFA date.”

Lumizyme (alglucosidase alfa) is a lysosomal glycogen-specific enzyme indicated for patients 8 years and older with late (non-infantile) onset Pompe disease (GAA deficiency) who do not have evidence of cardiac hypertrophy. The safety and efficacy of Lumizyme have not been evaluated in controlled clinical trials in infantile-onset patients, or in late (non-infantile) onset patients less than 8 years of age.

Genzyme began work on a therapy for Pompe disease ten years ago, and the company has invested nearly \$1 billion to support the development program. In 2006, Genzyme received approval for Myozyme® (alglucosidase alfa) in Europe and in other countries outside of the United States manufactured at a 2000 L bioreactor scale and indicated to treat all patients with Pompe disease. At this time, Genzyme also received FDA approval for Myozyme manufactured at a smaller 160 L bioreactor scale in the United States., which, because of its limited capacity, has been reserved for children and infants in the United States. In 2009, Genzyme received approval outside of the United States. for manufacturing Myozyme in 4000 L bioreactors at its state-of-the-art manufacturing facility in Geel, Belgium, and began to transition patients globally to the product manufactured at this larger scale. To prepare for growing demand for alglucosidase alfa, Genzyme has installed a third 4000 L bioreactor in Geel with an anticipated approval in 2011.

Genzyme has worked closely with patients and physicians in the U.S. Pompe community during the preapproval period to assure that the most severely affected late-onset patients could access therapy in advance of Lumizyme approval. In May 2007, Genzyme began providing alglucosidase alfa free-of-charge to patients in the United States through a program known as the Alglucosidase Alfa Temporary Access Program (ATAP). Nearly 200 severely affected adults in the United States. with Pompe disease are currently receiving treatment under the ATAP program. Genzyme will now work closely with the treating centers and prescribers to insure that patients in the ATAP program can continue to access therapy during the transition to

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commercial supply. Genzyme will also begin working with U.S. healthcare professionals to help adult patients who have been waiting to access treatment. In effort to preserve 160-L scale product for infantile-onset patients, Genzyme will begin to transition eligible patients who are receiving Myozyme onto Lumizyme.

Because Genzyme will market two approved alglucosidase alfa products in the United States, a Risk Evaluation and Mitigation Strategy called the Lumizyme ACE (alglucosidase alfa control and education) Program will be implemented for Lumizyme to ensure appropriate use for the intended patient populations. All prescribers of Lumizyme, and healthcare facilities where Lumizyme will be dispensed and administered, are required to be certified and enrolled in the Lumizyme ACE Program prior to treating patients with Lumizyme. Prescribers must also ensure patients enroll in the Lumizyme program prior to receiving therapy. Genzyme will begin this process immediately to certify and enroll prescribers and healthcare facilities and to help prescribers to enroll all patients that they intend to treat with Lumizyme.

About Pompe Disease

Pompe Disease is a progressively debilitating disease that 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.

Important Safety Information about Lumizyme

Lumizyme has a boxed warning as follows: Life-threatening anaphylactic reactions, severe allergic reactions and immune mediated reactions have been observed in some patients during Lumizyme infusions. Therefore, appropriate medical support should be readily available when Lumizyme is administered.

Because of the potential risk of rapid disease progression in Pompe disease patients less than 8 years of age, Lumizyme is available only through a restricted distribution program called the Lumizyme ACE Program. Only prescribers and healthcare facilities enrolled in the program may prescribe, dispense, or administer Lumizyme. Lumizyme may be administered only to patients who are enrolled in and meet all the conditions of the Lumizyme ACE Program. To enroll in the Lumizyme ACE Program call 1-800-745-4447.

Severe cutaneous reactions have been reported with alglucosidase alfa including necrotizing skin lesions. Systemic immune mediated reactions, including possible type III immune complex-mediated reactions have been observed with alglucosidase alfa. Patients with acute underlying respiratory illness or compromised cardiac and/or respiratory function may be at risk of serious exacerbation of their cardiac or respiratory compromise during infusions. Other serious adverse events that occurred in a higher incidence in Lumizyme treated patients compared to placebo included coronary artery disease, intervertebral disc protrusion, pneumonia, gastroenteritis, and dehydration. The most common adverse reactions observed in clinical studies were infusion reactions. Those occurring in Lumizyme treated patients at an incidence of $\geq 5\%$ compared to placebo included anaphylaxis, urticaria, diarrhea, vomiting, dyspnea, pruritus, rash/erythema,

pharyngolaryngeal pain, neck pain, hypoacusis, flushing/feeling hot, pain in extremity, fall and chest discomfort.

In post-marketing experience with Lumizyme, deaths, and serious adverse reactions have been reported, including anaphylaxis. Adverse events resulting in death reported in the postmarketing setting with Lumizyme treatment included cardiorespiratory arrest, respiratory failure, hemothorax, pneumothorax, cardiac failure, sepsis, aortic dissection, cerebrovascular accident, and skin necrosis. The most frequently reported serious adverse reactions were infusion reactions. The following serious adverse events have been reported in at least 2 patients: dyspnea, respiratory failure, bronchospasm, stridor, decreased oxygen saturation/hypoxia, pharyngeal edema, chest discomfort, chest pain, hypotension, hypertension, erythema, flushing, lung infection, tachycardia, cyanosis, and hypersensitivity. One case of hyperparathyroidism has been reported.

Please [click here](#) for the full prescribing information.

Important information safety information about Myozyme

Myozyme has a boxed warning as follows: Risk of Anaphylaxis

Life-threatening anaphylactic reactions have been observed in some patients during Myozyme infusions. Therefore, appropriate medical support should be readily available when Myozyme is administered.

Risk of Cardiorespiratory Failure

Patients with compromised cardiac or respiratory function may be at risk of serious acute exacerbation of their cardiac or respiratory compromise due to infusion reactions, and require additional monitoring.

Life-threatening and severe allergic reactions have included anaphylactic shock, cardiac arrest, respiratory distress, hypotension, bradycardia, hypoxia, bronchospasm, throat tightness, dyspnea, angioedema, and urticaria. Acute cardiorespiratory failure requiring intubation and inotropic support has been observed up to 72 hours after infusion with Myozyme in infantile-onset Pompe disease patients with underlying cardiac hypertrophy, possibly associated with fluid overload with intravenous administration of Myozyme. Caution should be used when administering general anesthesia for the placement of a central venous catheter in infantile-onset Pompe disease patients with cardiac hypertrophy. The most common adverse reactions requiring intervention were infusion-related reactions which occurred in 20 of 39 (51%) of patients treated with Myozyme in clinical studies. Some reactions were severe.

Please [click here](#) for the full prescribing information.

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. Since 1981, the company has grown from a small start-up to a diversified enterprise with more than 12,000 employees in locations spanning the globe and 2009 revenues of \$4.5 billion.

With many established products and services helping patients in approximately 100 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 disease, and diagnostic testing.

Genzyme's commitment to innovation continues today with a substantial development program focused on these fields, as well as cardiovascular disease, neurodegenerative diseases, and other areas of unmet medical need.

Genzyme's press releases and other company information are available at www.genzyme.com and by calling Genzyme's investor information line at 1-800-905-4369 within the United States or 1-678-999-4572 outside the United States.

This press release contains forward-looking statements regarding Genzyme's financial outlook and business plans including, without limitation, statements about the availability of Lumizyme and the potential expansion of manufacturing capacity for Myozyme and Lumizyme in Geel, Belgium. These statements are subject to risks and uncertainties that could cause actual results to differ materially from those forecasted. These risks and uncertainties include, among others: whether Genzyme has forecasted anticipated product demand accurately; whether Genzyme is able to manufacture product in sufficient quantities to meet demand; the timing of the implementation of the Lumizyme ACE Program; whether the FDA and other regulatory authorities approve the expansion of manufacturing capacity in Geel and the timing thereof; and the risks and uncertainties described in Genzyme's SEC reports filed under the Securities Exchange Act of 1934, including the factors discussed under the caption "Risk Factors" in Genzyme's Amended Quarterly Report on Form 10-Q/A for the quarter ended March 31, 2010. 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 these statements.

Genzyme[®] and Myozyme[®] are registered trademarks, and Lumizyme[™] is a trademark, of Genzyme Corporation or its subsidiaries. All rights reserved.

Important Information

On April 26, 2010, Genzyme filed a definitive proxy statement with the SEC in connection with the company's 2010 annual meeting of shareholders. Genzyme shareholders are strongly advised to read carefully the company's definitive proxy statement and other proxy materials before making any voting or investment decision because the definitive proxy statement and other proxy materials contain important information. The company's definitive proxy statement and any other reports filed by the company with the SEC can be obtained free of charge at the SEC's web site at www.sec.gov or from Genzyme at www.genzyme.com. Copies of the company's definitive proxy statement and other proxy materials are available for free by writing to Genzyme Corporation, 500 Kendall Street, Cambridge, MA 02142. In addition, copies of the proxy materials may be requested from our proxy solicitor, Innisfree M&A Incorporated, 501 Madison Avenue, 20th Floor, New York, NY 10022, toll free at: (888) 750-5835.

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