

PRESS RELEASE



For Immediate Release  
August 1, 2014

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## **Genzyme Receives Label Expansion for Lumizyme® (alglucosidase alfa) in the United States for the treatment of Pompe Disease**

CAMBRIDGE, Mass. – Genzyme, a Sanofi company (EURONEXT: SAN and NYSE: SNY), today announced that the U.S. Food and Drug Administration (FDA) approved a supplement to expand the indication for Lumizyme® (alglucosidase alfa). Lumizyme manufactured at the 4000L scale is now indicated for all Pompe patients of any age or phenotype. The approval of this indication is now consistent with that of the rest of the world, where alglucosidase alfa manufactured at the 4000L is the only scale available. Previously, in the United States, Lumizyme had been approved only for patients with late onset Pompe disease.

“We are thankful to the entire Pompe community who has been on this journey to provide a sustainable, long-term option for Pompe patients in the United States,” said Genzyme President and CEO, David Meeker, M.D. “We are pleased we can now offer alglucosidase alfa produced at the 4000L scale to all patients in the US.”

In the United States, alglucosidase alfa is manufactured at two different production scales. Alglucosidase alfa manufactured at the 160L scale (initial pilot scale) has a brand name of Myozyme® (alglucosidase alfa) and alglucosidase alfa manufactured at the 4000L (final manufacturing scale) has a brand name of Lumizyme.

Based on the biochemical and clinical data provided as part of the submission, FDA concluded that alglucosidase alfa manufactured at both scales in the US (i.e., Lumizyme and Myozyme) are comparable. The overall safety profile of alglucosidase alfa remains unchanged.

Specific updates to the Lumizyme product label include:

- Updated Indication: LUMIZYME® (alglucosidase alfa) is a hydrolytic lysosomal glycogen-specific enzyme indicated for patients with Pompe disease (GAA deficiency).
- Inclusion of safety and efficacy data from infantile-onset studies
- Removal of the REMS program
- Update to the boxed warning to include infantile-onset specific warning regarding fluid overload.
- Updated to Pregnancy Category C classification

Lumizyme, marketed as Myozyme outside of U.S., is approved in more than 65 countries. For the complete Lumizyme label, visit [www.lumizyme.com](http://www.lumizyme.com).

### About Pompe Disease

Pompe disease is a progressive, debilitating and often fatal neuromuscular disease caused by a genetic deficiency or dysfunction of the lysosomal enzyme acid alpha-glucosidase (GAA) affecting an estimated 1 in 40,000 people worldwide. This enzymatic defect results in the accumulation of glycogen primarily in muscle tissues that leads to muscle weakness, loss of respiratory function, and often premature death. Absent treatment, when symptoms occur in infancy, babies typically die within the first year of life. When symptoms occur in childhood or adulthood, patients often lose their ability to walk and require wheelchairs to assist with mobility and experience difficulty breathing as well as mechanical ventilation to breathe.

### Important Safety Information for Lumizyme and Myozyme

#### INDICATION

LUMIZYME® (alglucosidase alfa) is a hydrolytic lysosomal glycogen-specific enzyme indicated for patients with Pompe disease (acid  $\alpha$ -glucosidase (GAA) deficiency).

#### IMPORTANT SAFETY INFORMATION

**WARNING: RISK OF ANAPHYLAXIS, HYPERSENSITIVITY AND IMMUNE-MEDIATED REACTIONS, and RISK OF CARDIORESPIRATORY FAILURE**

***See full prescribing information for complete boxed warning.***

**Life-threatening anaphylactic reactions and severe hypersensitivity reactions, presenting as respiratory distress, hypoxia, apnea, dyspnea, bradycardia, tachycardia, bronchospasm, throat tightness, hypotension, angioedema (including tongue or lip swelling, periorbital edema, and face edema), and urticaria, have occurred in some patients during and after alglucosidase alfa infusions. Immune-mediated reactions presenting as proteinuria, nephrotic syndrome, and necrotizing skin lesions have occurred in some patients following alglucosidase alfa treatment. Closely observe patients during and after alglucosidase alfa administration and be prepared to manage anaphylaxis and hypersensitivity reactions. Inform patients of the signs and symptoms of anaphylaxis, hypersensitivity reactions, and immune-mediated reactions and have them seek immediate medical care should signs and symptoms occur.**

**Infantile-onset Pompe disease patients with compromised cardiac or respiratory function may be at risk of serious acute exacerbation of their cardiac or respiratory compromise due to fluid overload, and require additional monitoring.**

## **WARNINGS AND PRECAUTIONS**

**Anaphylaxis and Hypersensitivity Reactions:** Life-threatening anaphylaxis and hypersensitivity reactions have been observed in some patients during and after treatment with alglucosidase alfa. If anaphylaxis or severe hypersensitivity reactions occur, immediately discontinue infusion and institute appropriate medical treatment.

**Immune-Mediated Reactions:** Monitor patients for the development of systemic immune-mediated reactions involving skin and other organs.

**Risk of Acute Cardiorespiratory Failure:** Patients with compromised cardiac or respiratory function may be at risk of acute cardiorespiratory failure. Caution should be exercised when administering alglucosidase alfa to patients susceptible to fluid volume overload.

**Risk of Cardiac Arrhythmia and Sudden Cardiac Death during General Anesthesia for Central Venous Catheter Placement:** Caution should be used when administering general anesthesia for the placement of a central venous catheter intended for alglucosidase alfa infusion.

Appropriate medical support and monitoring measures should be available during infusion.

## **ADVERSE REACTIONS**

The most frequently reported adverse reactions ( $\geq 5\%$ ) in clinical trials were hypersensitivity reactions and included: anaphylaxis, rash, pyrexia, flushing/feeling hot, urticaria, headache, hyperhidrosis, nausea, cough, decreased oxygen saturation, tachycardia, tachypnea, chest discomfort, dizziness, muscle twitching, agitation, cyanosis, erythema, hypertension/increased blood pressure, pallor, rigors, tremor, vomiting, fatigue, and myalgia (6.1).

## **USE IN SPECIFIC POPULATIONS**

Pregnancy: Based on animal data, alglucosidase alfa may cause fetal harm.

**To report SUSPECTED ADVERSE REACTIONS, contact Genzyme at 1-800-745-4447 or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch)**

### **About Genzyme, a Sanofi Company**

Genzyme has pioneered the development and delivery of transformative therapies for patients affected by rare and debilitating diseases for over 30 years. We accomplish our goals through world-class research and with the compassion and commitment of our employees. With a focus on rare diseases and multiple sclerosis, we are dedicated to making a positive impact on the lives of the patients and families we serve. That goal guides and inspires us every day.

Genzyme's portfolio of transformative therapies, which are marketed in countries around the world, represents groundbreaking and life-saving advances in medicine. As a Sanofi company, Genzyme benefits from the reach and resources of one of the world's largest pharmaceutical companies, with a shared commitment to improving the lives of patients. Learn more at [www.genzyme.com](http://www.genzyme.com).

Genzyme<sup>®</sup>, Myozyme<sup>®</sup>, and Lumizyme<sup>®</sup> are registered trademarks of Genzyme Corporation. All rights reserved.

### **About Sanofi**

Sanofi, a global and diversified healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs. Sanofi has core strengths in the field of healthcare with seven growth platforms: diabetes solutions, human vaccines, innovative drugs, consumer healthcare, emerging markets, animal health and the new Genzyme. Sanofi is listed in Paris (EURONEXT: SAN) and in New York (NYSE: SNY).

### **Sanofi Forward Looking Statements**

*This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. These statements include projections and estimates and their underlying assumptions, statements regarding plans, objectives, intentions and expectations with respect to future financial results, events, operations, services, product development and potential, and statements regarding future performance. Forward-looking statements are generally identified by the words "expects", "anticipates", "believes", "intends", "estimates", "plans" and similar expressions. Although Sanofi's management believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Sanofi, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, the uncertainties inherent in research and development, future clinical data and analysis, including post marketing, decisions by regulatory authorities, such as the FDA or the EMA, regarding whether and when to approve any drug, device or biological application that may be filed for any such product candidates as well as their decisions regarding labelling and other matters that could affect the availability or commercial potential of such product candidates, the absence of guarantee that the product candidates if approved will be commercially successful, the future approval and commercial success of therapeutic alternatives, the Group's ability to benefit from external growth opportunities, trends in exchange rates and prevailing interest rates, the impact of cost containment policies and subsequent changes thereto, the average number of shares outstanding as well as those discussed or identified in the public filings with the SEC and the AMF made by Sanofi, including those listed under "Risk Factors" and "Cautionary Statement Regarding Forward-Looking Statements" in Sanofi's annual report on Form 20-F for the year ended December 31, 2013. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.*

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