Genzyme Study of Myozyme Treatment for Pompe Disease Published in New England Journal of Medicine

Release Date:
Thursday, April 15, 2010 9:04 am EDT

Terms:

Dateline City:
CAMBRIDGE, Mass.

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Genzyme Corp. (NASDAQ: GENZ) today announced that the results from its Late-Onset Treatment Study (LOTS) have been published in today's New England Journal of Medicine. The study was undertaken to evaluate the safety and efficacy of Myozyme (alglucosidase alfa) in older children and adults with Pompe disease.

The LOTS study was a randomized, double-blind, multi-national, placebo-controlled clinical trial that enrolled 90 older children and adults with Pompe disease. The study met its co-primary endpoints, demonstrating a beneficial effect of alglucosidase alfa on functional endurance and musculoskeletal strength as measured by the six-minute walk test, and on pulmonary function as measured by percent predicted forced vital capacity. Secondary endpoints of the study were supportive of these positive results.

"The data from LOTS demonstrated a positive effect of enzyme replacement therapy in older children and adults with Pompe disease," said Ans van der Ploeg, M.D., Ph.D., of Erasmus Medical Center in Rotterdam and the study's lead author. "The study is an important contribution to the field of Pompe disease."

About Myozyme

Myozyme was first approved in 2006, and is the only approved treatment for Pompe disease. In Europe, Myozyme is indicated for infants, children and adults and the prescribing information has recently been updated to include data from LOTS. In the U.S., Myozyme is also indicated for all ages but is only approved at a smaller production scale and is reserved for infants and children. Myozyme distributed in the U.S. 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. Genzyme is seeking FDA approval for alglucosidase alfa with the clinical data from LOTS for use in older children and adults produced at a larger production scale, to be known as Lumizyme. Genzyme has received an FDA action date of June 17, 2010 for Lumizyme.

About Pompe Disease

Pompe disease is a rare, neuromuscular disorder 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 and tissue involvement. When symptoms appear within a few weeks to months after birth, babies frequently display a markedly enlarged heart and unless treated die within the first year of life. When symptoms appear during childhood, adolescence or adulthood, patients may experience steadily progressive debilitation and unless treated may suffer premature mortality due to respiratory failure. Many patients often require mechanical ventilation to assist with breathing and wheelchairs to assist with mobility.

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.

Genzyme® is a registered trademark of Genzyme Corporation. All rights reserved.