FDA Approves New Treatment for Late-Onset Pompe Disease

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SILVER SPRING, Md., May 25 /PRNewswire-USNewswire/ -- The U.S. Food and Drug Administration approved Lumizyme (alglucosidase alfa) for patients ages 8 years and older with late-onset (non-infantile) Pompe disease, a rare genetic disorder.

(Logo: http://www.newscom.com/cgi-bin/prnh/20090824/FDALOGO [1])

Pompe disease occurs in an estimated 1 in every 40,000 to 300,000 births. Its primary symptom is heart and skeletal muscle weakness, progressing to respiratory weakness and death from respiratory failure.

In Pompe disease, a gene mutation prevents the body from making an enzyme, or making enough of the enzyme called acid alpha-glucosidase (GAA), necessary for proper muscle functioning. GAA is used by the heart and muscle cells to convert a form of sugar called glycogen into energy. Without the enzyme action, glycogen builds up in the cells and, ultimately, weakens the heart and muscles.

Lumizyme is believed to work by replacing the deficient GAA, thereby reducing the accumulated glycogen in heart and skeletal muscle cells.

"Pompe disease is a devastating condition without the appropriate treatment," said Julie Beitz, M.D., director of the Office of Drug Evaluation III in FDA's Center for Drug Evaluation and Research. "The approval of Lumizyme will provide an important treatment for patients diagnosed later in life with Pompe disease."

Lumizyme is being approved with a risk evaluation and mitigation strategy (REM
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SOURCE [2]

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