Update on Genzyme's neoGAA Program
July 16, 2013

Genzyme, a Sanofi Company, is moving into clinical development for a second generation product for Pompe disease (glycogen storage disease type II or acid maltase deficiency) known as neoGAA.

The purpose of this phase 1 study, called NEO1, is to investigate a second generation product for Pompe disease in humans for the first time. A phase 1 study is a very early study in the development of a new therapy. It is intended to assess safety parameters such as tolerability, pharmacokinetics (how the body processes a drug), and pharmacodynamics (the effect of a drug on the functions of the body).

The study is planned to be conducted at multiple sites in the United States and throughout Europe. This study will last up to 41 weeks and approximately 21 patients will participate in the study in two groups:

- 9 patients who have never received alglucosidase alfa therapy
- 12 patients who have received alglucosidase alfa for at least 9 months

Participants in the NEO1 study must be diagnosed with late-onset Pompe disease; be 18 years of age or older; be able to walk 50 meters (approximately 160 feet) without stopping and without an assistive device; and be able to have an MRI examination.

Additional information on this study will be available online at www.clinicaltrials.gov or https://www.clinicaltrialsregister.eu/. If you have further questions about this study, please contact your treating physician.