Update from Genzyme on neoGAA Pompe clinical development program 11th February 2014

Genzyme, a Sanofi company, has a long history of involvement with Pompe disease and partnership with the community. This led to the availability of the first approved treatment, enzyme replacement therapy (ERT) in 2006. We recognize that unmet medical need doesn't always mean developing a therapy for a disease with no treatment options, but can also mean improving upon an already existing treatment in a significant way. With the knowledge we gained during the development of an enzyme replacement therapy (ERT), Genzyme is particularly well equipped to attempt to provide a next-generation treatment for Pompe disease.

Genzyme is in the early stages of investigating a next generation enzyme replacement therapy (neoGAA) for Pompe disease. It differs from the original ERT, whereby carbohydrates are bonded onto the enzyme during the complex manufacturing process of neoGAA. These carbohydrates are thought to help the enzyme enter the muscles more efficiently. Though it's still very early in the development process and there are many unknowns, the hope is that through this more efficient uptake into the muscle cell, neoGAA might improve on the effectiveness of the existing therapy.

Currently there is an ongoing Phase I study known as NEO1, to evaluate the safety and efficacy of neoGAA dosing in adults with late-onset Pompe disease. A Phase I study is a very early study stage in the development of a new therapy. It is intended to measure safety parameters such as tolerability, pharmacokinetics (how the body processes the drug), and pharmacodynamics (the effect of the drug on the functions of the body.)

The study is planned to enroll 21 adult patients with late-onset Pompe disease, and will include patients who have never been treated with ERT and patients who have been previously treated with ERT for at least 9 months. NEO1 is designed to evaluate the safety and tolerability of 3 different doses of neoGAA (5 mg/kg, 10 mg/kg and 20 mg/kg) given by intravenous (IV) infusion once every 2 weeks for a total of 24 weeks. The study is being conducted at 18 sites in the United States and Europe (UK, Netherlands, Belgium, Denmark, France, and Germany). For more information please ask your treating physician or visit www.clinicaltrials.gov (ClinicalTrials.gov identifier: NCT01898364) or in Europe visit https://www.clinicaltrialsregister.eu EudraCT number 2012-004167-42

When the Phase I study is completed (2015), Genzyme will analyze the data to inform the next potential steps in this drug development program. Once we have significant updates on the neoGAA program, we will update the Pompe community accordingly.