Pompe Program Update May 2007

There are two studies to further evaluate Myozyme® (alglucosidase alfa) that will be conducted as part of Genzyme's postmarketing commitments (PMC) to the FDA. A postmarketing commitment is an agreement between a regulatory agency and a drug's sponsor to gather more information about a drug. While early clinical trials for Myozyme were used to provide safety and efficacy data to the FDA in support of the product's marketing approval, clinical studies are often performed as postmarketing commitments after a product's approval to help provide additional data about a drug's safety, efficacy, and optimal use. A postmarketing commitment can simply be a requirement to complete research that is currently underway, or to acquire and evaluate new information via a study or alternative mechanism. A clinical study that begins after a product's approval is also referred to as a phase 4 (IV) study.

The first Myozyme postmarketing study is AGLU03606, *A Long Term Study on Growth and Development Outcomes in Patients Less Than 1 Year of Age at the Time of Their First Myozyme Infusion.* The purpose of this 10-year study is to prospectively collect long-term growth and development outcomes data on newly diagnosed study participants with Pompe disease who are to be treated with Myozyme prior to their first birthday. The study includes assessments of physical growth, development, neuro-imaging, antibody response, and auditory and visual function at specific intervals designated in the study protocol over a 10-year period. Certain baseline assessments will be conducted within the trial before the study participant's first Myozyme infusion. Enrollment for this study began in early 2007 and will remain open to an unlimited number of participants until December 31, 2007.

The second study, AGLU03306, *A Dose and Dose Interval Exploratory Study in Patients Who Have Demonstrated Poor Responses to Myozyme or Have Sub-Optimal Improvement After a Minimum of 6 Continuous Months of Treatment With Myozyme at 20mg/kg Every Other Week and Who Are Currently Receiving Myozyme*, is being performed to explore whether or not changing the dose or frequency of administration of Myozyme can elicit a change in clinical response. Study participants will be randomized to either a weekly dose of 20mg/kg of Myozyme or a bi-weekly dose of 40mg/kg of Myozyme. Pompe patients 6 months of age or older, who meet the inclusion/exclusion criteria of the study, may be eligible. This study is 1 year in duration, and enrollment is open through January 2008; however, the study is limited to 12 participants.

At this time, both studies will be conducted in the United States as well as other specific countries as decided upon by Genzyme's clinical research team. To find out additional information about these trials, please refer to www.clinicaltrials.gov or have your physician contact Genzyme Medical Information at medinfo@genzyme.com or 1-800-745-4447.