

Update from Genzyme: Completion of Phase 1 Clinical Trial

Genzyme is pleased to announce that its Phase 1 clinical trial of recombinant human acid sphingomyelinase (rhASM) as a potential treatment for Acid Sphingomyelinase Deficiency (ASMD, Niemann-Pick Disease Type B) was completed in April 2009. The main purpose of this trial was to evaluate the safety of different doses of rhASM in adults with ASMD. A total of eleven patients were treated with single doses of rhASM ranging from 0.03 mg/kg up to 1 mg/kg administered intravenously. The trial took place at Mt. Sinai Medical Center in New York City.

The results of this trial have given an indication of the best ways to administer the drug intravenously to patients and monitor effectively for potential side effects. Genzyme is completing the Phase 1 data analyses and preparing for a Phase 2 trial that is expected to begin in 2010 and will likely involve giving repeat doses of rhASM. Genzyme plans to disseminate key findings from the Phase 1 trial as they become available.

Completion of the Phase 1 trial marks an important, hard-earned, and long-awaited milestone for the Niemann-Pick B disease community. Our sincere appreciation goes to all the patients who participated in the trial, and their families. Genzyme thanks Drs. McGovern and Wasserstein at Mt. Sinai for their leadership of the trial and Jessica Cristian and Erin Starrett for managing data collection activities.

Congratulations to the Niemann-Pick community on achieving this important milestone.

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