



**Update from Genzyme on  
Acid Sphingomyelinase Deficiency (ASMD) Development Efforts**  
December 9<sup>th</sup>, 2014

Genzyme, a Sanofi company, is pleased to update the Niemann-Pick disease patient community on the progress of continuing efforts to develop a potential therapy for acid sphingomyelinase deficiency (ASMD, also known as Niemann-Pick disease Types A and B).

We are actively preparing for a Phase 1/2 multi-center, open-label clinical trial in pediatric patients to evaluate the safety and tolerability of recombinant human acid sphingomyelinase (rhASM) when administered once every two weeks for 52 weeks. The trial is planned to enroll twelve pediatric patients up to 18 years of age with Niemann Pick Type B. Each participant in the trial is expected to receive rhASM once every two weeks, beginning at a low dose (0.03 mg/kg dose) and gradually increasing to a maximum dose of 3 mg/kg. No sites have been activated yet.

For more information about this clinical trial, please visit [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov) and refer to study reference number: NCT02292654

In addition to this Phase 1/2 pediatric trial, Genzyme also continues to prepare for a Phase 2/3 clinical trial in adult patients with Niemann Pick Type B to evaluate the safety and efficacy of different doses of rhASM when administered once every two weeks. We will provide another update when we have confirmed a start date for the Phase 2/3 clinical trial.

Genzyme remains committed to the Niemann-Pick community and will keep you updated as our development program continues. Genzyme extends its sincere gratitude to the patients and clinical sites that participated in the phase 1b clinical trial and are now enrolled in the open label study, whose dedication and commitment makes it possible to advance the understanding of this investigational therapy. We also thank the entire Niemann-Pick patient community for its ongoing support as we continue our efforts to develop a potential therapy for ASMD.

Please discuss any questions you may have with your treating physician.