



## **Update from Genzyme on Acid Sphingomyelinase Deficiency (ASMD) Development Efforts**

October 28, 2013

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

A Phase 1b clinical trial in Niemann-Pick Type B patients is ongoing to evaluate the safety and tolerability of an investigational enzyme replacement therapy recombinant human acid sphingomyelinase (rhASM). Five adult patients are enrolled in the trial at two study centers, Mount Sinai in New York, NY, US, and St. Mary's Hospital in Manchester, UK.

An infused protein-based product, rhASM, is being evaluated in this trial for the treatment of the non-neurological manifestations of ASMD. Each patient in the trial is receiving rhASM once every two weeks, beginning at a low dose (0.1 mg/kg dose) and gradually increasing to a maximum dose of 3 mg/kg. The trial will be completed in January, at which point the data will be reviewed and analyzed.

In addition to the ongoing Phase 1b clinical trial, we continue to make progress preparing for a Phase 2 trial to evaluate the safety and efficacy of different doses of rhASM when administered once every two weeks for one year. The Phase 2 trial is planned to start in Q1 2014. It will be a multi-center, international, 1-year trial of the safety and efficacy of rhASM in 15 adults with Niemann-Pick disease Type B. We plan to open clinical trial sites in the US, UK, Germany, Italy, France, Chile, Brazil, and potentially other countries. No sites have been activated yet.

On October 16<sup>th</sup>, Genzyme recognized Niemann-Pick Awareness Month by hosting two internal meetings for employees. At a breakfast meeting at Genzyme's research and development campus in Framingham, Massachusetts, employees heard three different stories of patients and their families living with Niemann Pick type B disease. Two of these stories were told through videos that patients made themselves and a third was told in-person by the mother and father of a little girl diagnosed with Niemann-Pick disease Type B earlier this year. Later that day employees at Genzyme's corporate headquarters in Cambridge, MA attended a similar presentation over lunch.

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During the two events, employees were presented with the need that exists around Niemann-Pick disease Type B and the hope that patients and families have for a better future.

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