SANOFI GENZYME

Phase 1/2 Olipudase Alfa Pediatric Study Enrollment Completed

We are pleased to let you know that the Phase 1/2 pediatric study evaluating the investigational therapy olipudase alfa has completed enrollment of all 12 patients. The Phase 1/2 study is a multi-national, multi-center, open label, ascending dose study to evaluate the safety, tolerability and pharmacokinetics of olipudase alfa administered intravenously once every 2 weeks for 64 weeks in pediatric patients with non-neurological ASMD, also known as Niemann-Pick disease types A and B. This study is specifically for Niemann-Pick type B. A total of twelve pediatric patients were enrolled into three age cohorts: an adolescent cohort (12 to <18 years of age); a child cohort (6 to <12 years of age); and an infant/early child cohort (birth to <6 years of age). The primary objective of the Phase1/2 study is to assess the safety and tolerability of olipudase alfa. Upon completion of the 64-week study, patients will have the option to enroll into the Long Term study. For more information please visit https://clinicaltrials.gov.

Sanofi Genzyme is currently conducting three clinical studies of olipudase alfa– a Phase 1/2 study in pediatric patients, a Phase 2/3 study in adult patients and a Long Term study. There are currently no approved treatment options for patients with Niemann-Pick disease types A and B.