Pivotal Phase 2/3 Trial of Olipudase Alfa for Adult Patients with Acid Sphingomyelinase Deficiency (Niemann-Pick Disease Type B) to Begin in the UK

Oxford, UK – 3 November, 2016 – Sanofi Genzyme, the specialty care global business unit of Sanofi, announced today that the first adult patient in the UK has enrolled and been dosed in a pivotal Phase 2/3 clinical trial named ASCEND for the investigational therapy olipudase alfa. Olipudase alfa is an enzyme replacement therapy being studied for the treatment of non-neurological manifestations of acid sphingomyelinase deficiency (ASMD), also known as Niemann-Pick disease type B (NPD B). The first patient was dosed at the National Hospital for Neurology and Neurosurgery at the University College of London Hospitals (UCLH).

"ASMD is a rare disorder affecting the breakdown of certain complex fats in the body," said Dr. Robin Lachmann, Principal Investigator at the National Hospital for Neurology and Neurosurgery. "These gradually accumulate over time in organs such as the liver, spleen and lungs, leading to debilitating and life threatening complications. There is a clear need for a new treatment option which could positively affect the lives of patients with ASMD. We are excited to be taking part in this pivotal clinical trial and pleased to have been able to dose the first patient in the UK."

"There is currently no approved treatment for ASMD and so today's announcement of the first patient in the UK receiving olipudase alfa in this trial is a very welcome step," said Toni Mathieson, Chief Executive at Niemann-Pick UK (NPUK). *"We look forward to seeing the trial progress and to continuing to work closely with physicians, patients and companies, such as Sanofi Genzyme, who are committed to developing effective treatments for rare diseases."*

ASMD is one of a group of lysosomal storage disorders that affect cellular metabolism and are caused by genetic mutations. ASMD is a serious and life-threatening disorder caused by insufficient activity of the enzyme acid sphingomyelinase resulting in accumulation of sphingomyelin in multiple organs of the body. Common clinical manifestations include enlarged liver and spleen, liver dysfunction, infiltrative lung disease, bleeding complications, cardiovascular and bone disease, and growth delay. There are currently no approved treatment options for patients with ASMD.

ASCEND is a Phase 2/3 multi-national, multi-center, double-blinded, placebo-controlled trial to evaluate the efficacy, safety, pharmacodynamics and pharmacokinetics of olipudase alfa administered intravenously once every 2 weeks for 52 weeks in adult patients with ASMD, specifically NPD B. The Phase 2/3 trial will assess the effect of olipudase alfa on spleen size, lung function and other important clinical parameters. Thirty-six patients are expected to be enrolled in the study and receive olipudase alfa or a placebo. Upon completion of the 52 week primary analysis period, all patients will receive treatment in an extension period.

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About Sanofi

Sanofi, a global healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs. Sanofi is organized into five global business units: Diabetes and Cardiovascular, General Medicines and Emerging Markets, Sanofi Genzyme, Sanofi Pasteur and Merial.

Sanofi Genzyme focuses on developing specialty treatments for debilitating diseases that are often difficult to diagnose and treat, providing hope to patients and their families.