Pivotal Phase 3 Trial of NeoGAA Investigational Second-Generation Therapy for Pompe Disease to Begin in the UK

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OXFORD, England, November 17, 2016 /PRNewswire/ --

Sanofi Genzyme, the specialty care global business unit of Sanofi, today announced that the first patient in the UK has been enrolled and received an infusion in a pivotal Phase 3 clinical trial named COMET for the investigational therapy neoGAA. NeoGAA is a second-generation enzyme replacement therapy being studied for the treatment of Pompe disease. The first patient was dosed at the University of Newcastle upon Tyne.

"Pompe disease is a serious, progressive and debilitating disorder," said Professor Volker Straub, Principal Investigator at the Newcastle University John Walton Muscular Dystrophy Research Centre and the Newcastle Hospitals NHS Foundation Trust. *"Dosing the first patient in the UK is an exciting and important milestone in our quest to advance treatment options for patients with this disease."*

"Today's news marks a long-awaited step in the continuing effort to develop new treatments for people living with Pompe disease," added Allan Muir, Charity Director of the Association for Glycogen Storage Disease UK. *"The UK patient community is proud to play such an important role in this global trial of a second generation enzyme replacement therapy and we look to forward to following its progress."*

Pompe disease is a progressive, debilitating and often fatal neuromuscular disease

Pompe disease is a progressive, debilitating and often fatal neuromuscular disease caused by a genetic deficiency or dysfunction of the lysosomal enzyme acid alpha-glucosidase (GAA) affecting an estimated 50,000 people worldwide. Patients often lose their ability to walk and require wheelchairs to assist with mobility. They also often experience difficulty breathing and may require mechanical ventilation to breathe.

COMET is a Phase 3 randomised, multi-center, multi-national, double-blinded study to compare the efficacy and safety of repeated bi-weekly infusions of neoGAA and alglucosidase alfa in treatment-naïve patients with late-onset Pompe disease. The primary endpoint of the Phase 3 trial is the effect of neoGAA on respiratory muscle strength as measured by percent predicted forced vital capacity in the upright position. Other assessments include functional endurance measured by the 6 minute walk test, muscle strength, motor function, health-related quality of life, and patient reported outcomes. Approximately 96 patients, ages 3 and up, are expected to be enrolled in the study, which will last up to 3 years, including a 49-week blinded treatment period and a 96-week open-label treatment period. For more information on the trial, please visit https://www.clinicaltrials.gov/
or https://www.clinicaltrialsregister.eu .

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.

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