Sanofi Genzyme Presents Results from Phase 1/2 Study of Investigational Second-Generation Therapy for Pompe Disease

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- **Results support beginning pivotal Phase 3 clinical trial in Q2 2016** -

PARIS--(BUSINESS WIRE)--Sanofi and its specialty care global business unit Sanofi Genzyme today presented data from NEO1, its Phase 1/2 clinical study evaluating the investigational novel enzyme replacement therapy neoGAA in 24 patients with late-onset Pompe disease. The safety and efficacy data from this study, which were presented at WORLD Symposium 2016 in San Diego, CA, support further development of the therapy. Sanofi Genzyme plans to begin enrolling patients in a pivotal Phase 3 trial for neoGAA in Q2 2016.

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.

**Study Design**
The NEO1 study was an open-label, multicenter, multinational, ascending dose study of safety, tolerability, pharmacokinetics, pharmacodynamics and exploratory efficacy of investigational neoGAA in treatment-naïve and alglucosidase alfa-treated (treatment-experienced) late-onset Pompe disease patients. Adult patients with acid α-glucosidase enzyme deficiency who could walk ≥50 m independently without stopping and had upright forced vital capacity (FVC) ≥50% predicted at baseline received intravenous infusion neoGAA (5, 10 or 20 mg/kg every other week) for 24 weeks. In the treatment-naïve group, 9 of 10 treated patients completed the study. In the treatment-experienced patient group, 12 of 14 treated patients completed the study.

**Safety Data**
NeoGAA was generally safe and well tolerated at all dose levels. There were no deaths or life-threatening serious adverse events (SAEs). One patient experienced a study drug related SAE of respiratory distress and chest discomfort and discontinued treatment. Two additional patients withdrew consent for non-AE related reasons. Overall, 8 of 10 patients (80.0%) in the treatment-naïve group and 12 of 14 patients (85.7%) in the treatment-experienced group had at least one treatment-emergent AE during the study. The majority of treatment-emergent AEs were non-serious, mild to moderate in intensity and assessed as unrelated to study drug. The most frequently reported treatment-emergent AEs considered related to study drug were myalgia or muscle pain (7 events in 2 patients), headache (3 events in 2 patients) and fatigue (3 events in 3 patients).

**Exploratory Efficacy**
No clear response relationship was observed between dose levels or treatment groups. At the highest dose tested, 20 mg/kg, which is the dose that will be used in the Phase 3 clinical trial, percent predicted FVC, maximal expiratory pressure (MEP) and maximal inspiratory pressure (MIP) increased by means (± SD) of 6.2 ± 3.2%, 12.0 ± 4.1%, and 7.9 ± 15.7%, respectively, from baseline to Week 25 in treatment-naïve patients; corresponding changes in treatment-experienced patients were 1.4 ± 5.7%, 6.0 ± 21.8%, and -0.2 ± 6.9%. After 24 weeks of treatment with the 20 mg/kg dose, the 6-minute walk test distance increased by 24.3 ± 23.0 m in treatment-naïve patients and decreased by 6.2 ± 64.3 m in treatment-experienced patients.

“Respiratory function measures are important clinical indicators for Pompe patients,” said Loren D.M. Pena, MD, PhD, Assistant Professor of Pediatrics, Duke University School of Medicine and coordinating investigator for the study. “The positive trending of exploratory data across all three respiratory endpoints -- percent predicted FVC, MEP and MIP -- suggests improvement or stabilization of pulmonary function in late-onset Pompe patients. This combined with the safety data indicates the potential for further development of neoGAA.”

“Sanofi Genzyme has a long history of investing in research and development to further the understanding of Pompe disease and advance treatment options,” said Jorge Insuasty, Head of Global Development.

“We are highly encouraged that the safety profile and exploratory efficacy assessments provide positive proof of concept to continue the clinical development of neoGAA, which we plan to undertake later this year with a Phase 3 clinical trial.”

**About neoGAA**
NeoGAA is an investigational second-generation alglucosidase alfa enzyme replacement therapy that has been specifically designed for enhanced receptor targeting and enzyme uptake through greater affinity for the M6P receptors on muscle cells, with the aim of enhancing glycogen clearance and improving on the clinical efficacy achieved with alglucosidase alfa. In preclinical studies, neoGAA showed approximately five-fold greater potency than alglucosidase alfa in terms of tissue glycogen reduction compared to alglucosidase alfa. In the Pompe mouse model, neoGAA reduced similar levels of substrate glycogen compared to alglucosidase alfa.
at one-fifth the dose of alglucosidase alfa. The clinical significance of this data requires further investigation.

About Sanofi
Sanofi, a global healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients’ needs. Sanofi has core strengths in diabetes solutions, human vaccines, innovative drugs, consumer healthcare, emerging markets, animal health and Genzyme. Sanofi is listed in Paris (Euronext: SAN) and in New York (NYSE: SNY).

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. Learn more at www.sanofigenzyme.com.

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Sanofi Forward-Looking Statements
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1 Zhu et al, Molecular Therapy, 2009.

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