



## Sanofi Genzyme Begins Pivotal Phase 3 Trial of NeoGAA Investigational Second-Generation Therapy for Pompe Disease

**Cambridge, Mass. – November 4, 2016** - [Sanofi Genzyme](#), the specialty care global business unit of [Sanofi](#), announced today that the first patient 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.

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 randomized, 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>.

*“The beginning of this pivotal trial is a critical milestone in Sanofi Genzyme’s long history of advancing the understanding of Pompe disease,”* said Sanofi Genzyme Therapeutic Area Head for Rare Diseases Development Rand Sutherland, M.D. *“We are committed to researching and developing novel treatment options to address the unmet needs of Pompe patients.”*

*“Pompe disease is a serious and progressive condition,”* said Shafeeq S. Ladha, M.D., Ira A. and Mary Lou Fulton Chair in Motor Neuron Diseases, Director, Gregory W. Fulton ALS and Neuromuscular Disorders Center, Department of Neurology, Barrow Neurological Institute. *“My hope is that a second generation enzyme replacement therapy with improved targeting to skeletal muscle may provide additional benefit to patients with this debilitating disease.”*

### **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 at one-fifth the dose of alglucosidase alfa.<sup>1</sup> The clinical significance of this data requires further investigation.

Results from the Phase 1/2 proof of concept study were reported at the WORLD Symposium earlier this year.

## 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 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.

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## Forward-Looking Statements

*This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. These statements include projections and estimates and their underlying assumptions, statements regarding plans, objectives, intentions and expectations with respect to future financial results, events, operations, services, product development and potential, and statements regarding future performance. Forward-looking statements are generally identified by the words "expects", "anticipates", "believes", "intends", "estimates", "plans" and similar expressions. Although Sanofi's management believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Sanofi, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, the uncertainties inherent in research and development, future clinical data and analysis, including post marketing, decisions by regulatory authorities, such as the FDA or the EMA, regarding whether and when to approve any drug, device or biological application that may be filed for any such product candidates as well as their decisions regarding labelling and other matters that could affect the availability or commercial potential of such product candidates, the absence of guarantee that the product candidates if approved will be commercially successful, the future approval and commercial success of therapeutic alternatives, Sanofi's ability to benefit from external growth opportunities and/or obtain regulatory clearances, risks associated with intellectual property and any related pending or future litigation and the ultimate outcome of such litigation, trends in exchange rates and prevailing interest rates, volatile economic conditions, the impact of cost containment initiatives and subsequent changes thereto, the average number of shares outstanding as well as those discussed or identified in the public filings with the SEC and the AMF made by Sanofi, including those listed under "Risk Factors" and "Cautionary Statement Regarding Forward-Looking Statements" in Sanofi's annual report on Form 20-F for the year ended December 31, 2015. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.*

<sup>1</sup> Zhu et al, Molecular Therapy, 2009.

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