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Sanofi Genzyme Begins Pivotal Phase 2/3 Trial of Olipudase Alfa for Adult Patients with Acid Sphingomyelinase Deficiency

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sanofi Genzyme, the specialty care global business unit of Sanofi, announced today that the first adult patient 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 nonneurological manifestations of acid sphingomyelinase deficiency (ASMD), also known as Niemann-Pick disease type B (NPD B).

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.

"*ASMD is a rare and debilitating disease that can lead to serious medical conditions including failure of the lungs, liver or heart,"* said Eugen Mengel, M.D., Principal Investigator at the Villa Metabolica Mainz University Medical Center. *"The beginning of this pivotal trial is a critical milestone in the assessment of olipudase alfa's potential to impact the lives of patients living with ASMD."*

In June of last year, Sanofi Genzyme announced the beginning of a Phase 1/2 trial in pediatric patients with ASMD, specifically NPD B. For more information on both trials, please visit https://www.clinicaltrials.gov/ or https://www.clinicaltrialsregister.eu. The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to olipudase alfa. Breakthrough Therapy designation is intended to expedite the development and review of investigational new drugs that target serious or life-threatening conditions.

"We are excited to have clinical studies for olipudase alfa now underway for both pediatric and adult patients," said Therapeutic Area Head, Rare Diseases Development Rand Sutherland, M.D. *"This is an important step forward in our journey to meet the need for a meaningful treatment option for patients with ASMD. We are thankful for the engagement and support of the patients, physicians and scientific community for helping us reach this milestone."*

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.

Sanofi 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, the Group's ability to benefit from external growth opportunities, trends in exchange rates and prevailing interest rates, 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.

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