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FDA Grants Breakthrough Therapy Designation for Genzyme's Olipudase Alfa

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Investigational enzyme replacement therapy designed to treat the nonneurological manifestations of ASMD which characterize Niemann-Pick disease type B

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Genzyme, a Sanofi company, announced today that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to olipudase alfa. This enzyme replacement therapy is being investigated for the treatment of patients with nonneurological manifestations of acid sphingomyelinase deficiency (ASMD), also known as Niemann-Pick disease type B, as opposed to type A which is characterized by neurological involvement. ASMD is a serious and life-threatening disorder caused by insufficient activity of the enzyme acid sphingomyelinase (ASM), which results in toxic accumulation of sphingomyelin. There are currently no approved treatment options for patients with Niemann-Pick disease type B.

Breakthrough Therapy designation is intended to expedite the development and review of investigational new drugs that target serious or life-threatening conditions. The criteria for granting Breakthrough Therapy designation are preliminary clinical evidence of substantial improvement on a clinically significant endpoint over available therapies. The Breakthrough Therapy designation is distinct from the FDA's other mechanisms to expedite drug development and review, and will allow for a close collaboration between Genzyme and the FDA on the olipudase alfa development program.

Olipudase alfa is being developed by Genzyme to potentially address the fundamental defect underlying the disease. Supplementing the defective or deficient native enzyme with olipudase alfa allows for the breakdown of sphingomyelin, whose accumulation is responsible for the clinical manifestation of ASMD.

The Breakthrough Therapy designation is supported by data from a completed Phase 1b study of olipudase alfa. Findings in five adult patients with nonneuronopathic ASMD were presented at the Lysosomal Disease Network's WORLD Symposium in February 2015. The data presented on the repeat-dose safety, pharmacodynamics, and exploratory efficacy of olipudase alfa support its continued development for the investigational use in nonneurological manifestations of ASMD.

The company has started enrollment of a Phase 1/2 pediatric study and is preparing for enrollment of a Phase 2/3 adult study in the second half of 2015. For more information please visit http://clinicaltrials.gov.

"There is tremendous unmet need in the ASMD/Niemann-Pick disease type B community, and we are hopeful that olipudase alfa can be developed into a meaningful treatment for patients," said Genzyme's Global Head of Rare Diseases, Richard Peters, MD, Ph.D. "We appreciate FDA's support for this important program giving us the opportunity to utilize an important expedited drug development pathway for olipudase alfa and providing hope for patients affected with a chronic and progressively debilitating disease."

About ASMD (Niemann-Pick Disease)

Traditionally called Niemann-Pick disease types A and B (NPD A and NPD B), Acid Sphingomyelinase Deficiency (ASMD) is one of a group of lysosomal storage diseases that affect the metabolism and that are caused by genetic mutations. ASMD is caused by the deficiency of a specific enzyme, acid sphingomyelinase (ASM). This enzyme is found in special compartments within cells called lysosomes and is required to metabolize a lipid called sphingomyelin. If ASM is absent or not functioning properly, sphingomyelin cannot be metabolized properly and is accumulated within the cell, eventually causing cell death and the malfunction of major organ systems. Niemann-Pick A and Niemann-Pick B are both caused by the same enzymatic deficiency and there is growing evidence that the two forms represent opposite ends of a continuum.

About Genzyme, a Sanofi Company

Genzyme has pioneered the development and delivery of transformative therapies for patients affected by rare and debilitating diseases for over 30 years. We accomplish our goals through world-class research and with the compassion and commitment of our employees. With a focus on rare diseases and multiple sclerosis, we are dedicated to making a positive impact on the lives of the patients and families we serve. That goal guides and inspires us every day. Genzyme's portfolio of transformative therapies, which are marketed in countries around the world, represents groundbreaking and life-saving advances in medicine. As a Sanofi company, Genzyme benefits from the reach and resources of one of the world's largest pharmaceutical companies, with a shared commitment to improving the lives of patients. Learn more at www.genzyme.com.

About Sanofi

Sanofi, a global and diversified healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs. Sanofi has core strengths in the field of healthcare with seven growth platforms: 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).

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 policies 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, 2014. 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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