Genzyme Presents Data from its Phase 1b Program for Niemann-Pick Type B at the Lysosomal Disease Network’s WORLD Symposium 2015

Release Date:
Thursday, February 12, 2015 4:15 pm EST

Terms:

Dateline City:
CAMBRIDGE, Mass.

Study showed tolerability of dose-escalation regimen

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Genzyme, a Sanofi company, today presented data from its Phase 1b clinical study at the Lysosomal Disease Network’s WORLD Symposium 2015 in Orlando, Fla. detailing the investigational use of enzyme replacement therapy in the non-neurological manifestations of acid sphingomyelinase deficiency (ASMD, also known as Niemann-Pick disease type B), a lysosomal storage disease caused by genetic mutations that affect the metabolism of sphingomyelin. The Genzyme study evaluated the tolerability and safety of olipudase alfa (recombinant human acid sphingomyelinase) in five adult patients with ASMD.

Melissa P. Wasserstein, MD, Director of the Program for Inherited Metabolic Diseases; Medical Director of the International Center for Types A and B Niemann Pick Disease, Mount Sinai School of Medicine, presented: An open-label, multicenter, ascending-repeat-dose study of the tolerability and safety of recombinant human acid sphingomyelinase (rhASM) in patients with ASM deficiency (ASMD). In the trial, each patient received a starting dose of intravenous olipudase alfa at 0.1 mg/kg and escalated dosing every two weeks according to a predetermined schedule up to 3 mg/kg or their maximum tolerated dose. The secondary objective was to study the pharmacokinetics, pharmacodynamics, and exploratory efficacy of olipudase alfa administered every two weeks for 26 weeks. The study findings showed that the dose escalation regimen was well tolerated, with all patients reaching the maximum dose of 3 mg/kg. No serious or severe adverse events or deaths were reported. The data presented on the repeat-dose safety, pharmacodynamics, and exploratory efficacy of olipudase alfa support its continued development for the investigational use in non-neurological manifestations of ASMD. All five patients are participating in the Long-Term Study and will continue on therapy.

“Though a small number of patients, the response we have observed to date is an early indication that this ASM enzyme replacement therapy is promising for this therapeutic area,” said Genzyme’s Acting Head of Rare Diseases, Richard Peters, M.D., Ph.D. “We look forward to continuing this program and learning more as we work toward advancing a treatment option for patients that is both safe and well tolerated.” Genzyme plans to begin enrolling patients in a Phase 2/3 program for Niemann-Pick Type B in 2015.

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.

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About Sanofi

Sanofi, a global 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, 2013. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.

Language:
English

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