Genzyme Collaborates on Gene Therapy for Rare Disease that Causes Childhood Blindness

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Genzyme to Provide $900,000 Grant to University of Florida Researcher

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Genzyme, a Sanofi company (EURONEXT: SAN and NYSE: SNY), today announced the establishment of a research collaboration with the University of Florida and the University of Pennsylvania to develop a gene therapy for the treatment of a rare genetic disease that causes childhood blindness. Leber congenital amaurosis type 1 (LCA-1) is usually diagnosed in children who are less than a year old, and patients remain severely visually impaired for the rest of their lives.

Genzyme is providing $900,000 in funding over three years to Dr. Shannon Boye, assistant professor of ophthalmology at the University of Florida, to fund her LCA-1 research. Dr. Boye’s research is focused on a gene called guanylate cyclase (GUCY2D) that is mutated in LCA-1 patients. The GUCY2D gene normally makes GC1, a protein expressed in photoreceptors, which are located in the retina of the eye and convert light into electrical signals that the brain interprets as vision.

Genzyme’s funding supplements current funding from the Foundation Fighting Blindness.

The majority of the early stage LCA-1 research is being conducted at the University of Florida, with Genzyme taking on increasingly more activities as the program advances toward clinical trials. Genzyme has the option to in-license the potential treatment before it enters clinical trials.

“Genzyme is a very collaborative partner and has been wonderful to work with,” said Dr. Boye. “We share a heartfelt commitment to helping LCA-1 patients and their families and to pursuing a treatment building upon prior, promising non-clinical studies.”

The gene therapy is administered by inserting a healthy copy of the GUCY2D gene into the eye. The healthy gene is put into an adeno associated viral (AAV) vector whose viral DNA has already been removed. The viral vector is injected into the retina and transfers the healthy gene to the photoreceptors, with the goal of treating the disease with a single treatment.

“This research is both very promising and very important,” said Abraham Scaria, Senior Scientific Director and Project Leader for the LCA-1 program. “We are excited by the prospect of what this treatment would mean for children diagnosed with this disease.”

Treating physicians of LCA-1 patients from the University of Pennsylvania are also playing a key role in this research. They provide information to the University of Florida and Genzyme about indicators to look for in trials to know whether or not the therapy is working. They are also conducting a natural history study, which tracks how the retina of an LCA-1 patient progresses over time if untreated.

“We are proud to partner with Dr. Shannon Boye and her team at the University of Florida, who are leading experts in the field of ocular gene therapy, as well as our clinical colleagues from the University of Pennsylvania, who are sharing important insights about LCA-1 patients’ needs,” said Rich Gregory, Ph.D., Head of the Sanofi Genzyme R&D Center. “Together, we are striving to provide new hope to children who might not otherwise be able to see.”

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 the new 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.

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Contact:
Genzyme
Lori Gorski, 617-768-9344
Lori.Gorski@genzyme.com

Ticker Slug:
Ticker: SNY
Exchange: NYSE
ISIN: US80105N1054

Ticker: SAN
Exchange: BOURSE
ISIN: FR0000120578