Sanofi Initiates Phase 2 Clinical Trial to Evaluate Therapy for Genetic Form of Parkinson’s Disease

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sanofi Genzyme, the specialty care global business unit of Sanofi, today announced the start of a Phase 2 trial of an investigational oral therapy for patients with Parkinson’s disease who carry a single copy of a GBA gene mutation that is the most common genetic risk factor for the disease. The trial will assess the drug’s dynamics, efficacy and safety. This is the first industry-sponsored Phase 2 clinical trial in a genetically defined population of Parkinson’s disease.

Parkinson’s disease is a chronic, degenerative neurological disorder affecting an estimated one million people in the United States and more than five million people worldwide. An estimated 5 – 10% of Parkinson's disease patients carry a mutation of the glucocerebrosidase (GBA) gene that allows lipids called glycosphingolipids to build up in cells. The molecule being studied, GZ/SAR402671, reduces the production of glycosphingolipids.

“Patients with Parkinson’s disease and a GBA gene mutation are predisposed to develop motor symptoms at a younger age, have a higher prevalence of cognitive impairment and undergo more rapid disease progression,” explains Anthony Schapira, MD, DSc, FRCP, FMedSci, Head of Department of Clinical Neurosciences, UCL Institute of Neurology and Lead Principal Investigator for the study. “Investigating a targeted therapy for these patients is an important first step in addressing the serious unmet needs these patients and their families face in managing Parkinson’s disease.”

The clinical trial, known as MOVES-PD, will be run in two phases: a dose escalation study followed by a study of efficacy and safety. The randomized, double blind study will enroll more than 200 patients at trial sites around the world. The primary endpoint of the study is the change in score from baseline in a scale commonly used to measure motor progression known as the Movement Disorder Society Unified Parkinson’s Disease Rating Scale Part II and III. This includes self-evaluation of daily life activities and motor experience, and a clinician-scored motor evaluation.

“We are excited to be able to bring the results of our many years of research in GBA gene mutations to a new therapeutic area with the potential to benefit patients with Parkinson’s disease,” said Tanya Fischer, MD, PhD, Global Project Head of Early Development for Parkinson’s Disease and Movement Disorders, Sanofi R&D. “We look forward to evaluating whether this molecule, which has been shown to cross into the brain in preclinical studies, may positively impact the devastating neurologic effects of this disease.”

Sanofi has studied GBA gene mutations for more than 30 years. People with GBA mutations in both copies of the gene, as opposed to a single mutation in GBA Parkinson’s disease, have Gaucher disease. Gaucher disease is a rare genetic disorder in which the buildup of a lipid in the cells leads to a broad spectrum of systemic manifestations including bruising, fatigue, anemia, low blood platelets, bone and joint pain, enlargement of liver and spleen, as well as neurological manifestations such as seizures and incoordination in severe forms.

Sanofi Genzyme introduced the world’s first treatment for Gaucher disease and Sanofi R&D remains committed to developing treatments for conditions associated with GBA mutations, including Gaucher disease and Parkinson’s disease.

For more information on this trial, please visit https://www.clinicaltrials.gov/ or https://www.clinicaltrialregister.eu.

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 Consumer Healthcare.

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, 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.

1 Michael J. Fox Foundation. 2016. Understanding Parkinson's [ONLINE]. Available at: https://www.michaeljfox.org/understanding-parkinsons/i-have-got-what.php [Accessed 8 December 2016]

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