Sanofi provides update on venglustat clinical program

PARIS – JUNE 1, 2021 – A pivotal Phase 2/3 study of venglustat in autosomal dominant polycystic kidney disease (ADPKD) did not meet futility criteria, and the company has halted the clinical program in ADPKD. The safety profile of venglustat remains consistent with previously reported results with more than 500 patients treated to date over a period of up to four years across all clinical programs. Biomarker data from the study confirmed venglustat effectively inhibits the glycosphingolipid (GSL) pathway by demonstrating a reduction in GL-1, a lipid that accumulates in certain cells.

The STAGED-PKD study was stopped for futility following an independent analysis of the annualized rate of change in total kidney volume (TKV) in patients receiving venglustat compared to placebo. Trends from the analysis showed venglustat did not provide a meaningful reduction in TKV growth rate, the primary endpoint of stage 1 of the Phase 2/3 study. This interim analysis suggests the reduction of GSLs may not play a significant role in the prevention of kidney cyst growth, and as such, may not be a primary pathway associated with the progression of ADPKD. The investigational research of venglustat in ADPKD was an attempt to explore a novel biological role for GSLs beyond the established role of these lipids in lysosomal storage diseases (LSDs).

“The venglustat development program started with our confidence in the promise of a potential breakthrough treatment to address the unmet needs of people living with lysosomal storage disorders,” said John Reed, M.D., Ph.D., Global Head of Research and Development at Sanofi. “In parallel, we set out to evaluate venglustat in autosomal dominant polycystic kidney disease, a leading cause of kidney transplant. This outcome is not what we hoped for, especially for these patients. However, our research has furthered the scientific understanding of ADPKD by demonstrating that modulating the GSL pathway is insufficient to restore kidney function in adults affected by this disease.”

Sanofi has both completed and active studies evaluating venglustat in Gaucher disease type 3, Fabry disease and GM2 Gangliosidosis. These diseases are LSDs caused by inherited genetic abnormalities. The abnormal accumulation of GSLs is central to certain LSDs and therapeutics promoting the clearing of these accumulated lipids have been validated in Fabry disease and Gaucher disease through clinical research. In this context, venglustat operates as a Substrate Reduction Therapy (SRT), which is a concept that has also been previously validated for certain LSDs.
About venglustat

GSLs are cellular building blocks whose abnormal accumulation is implicated in several rare diseases, responsible for both cell dysfunction and disease progression. Venglustat is a novel, oral investigational therapy that has the potential to slow the progression of certain diseases by inhibiting abnormal GSL accumulation. Venglustat is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority.

About Sanofi

Sanofi is dedicated to supporting people through their health challenges. We are a global biopharmaceutical company focused on human health. We prevent illness with vaccines, provide innovative treatments to fight pain and ease suffering. We stand by the few who suffer from rare diseases and the millions with long-term chronic conditions.

With more than 100,000 people in 100 countries, Sanofi is transforming scientific innovation into healthcare solutions around the globe.

Sanofi, Empowering Life

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