Press Release

Sanofi's venglustat met all primary endpoints in a phase 3 study of type 3 Gaucher disease

- In the LEAP2MONO phase 3 study, venglustat, dosed orally once daily, demonstrated clinically meaningful efficacy in patients with type 3 Gaucher disease (GD3), a rare lysosomal storage disorder
- Venglustat demonstrated superiority versus enzyme replacement therapy in addressing neurological symptoms in GD3, for which there are no approved treatments
- Sanofi will pursue global regulatory submissions for GD3
- In the PERIDOT phase 3 study in Fabry disease, venglustat did not show superiority on the patient-reported primary endpoint and an additional phase 3 CARAT study is ongoing

Paris, February 2, 2026. Positive results from the LEAP2MONO phase 3 study (clinical study identifier: NCT05222906) demonstrated that venglustat met the primary and three out of four key secondary endpoints in adults and pediatric patients (12 years and older) with neurological manifestations of type 3 Gaucher disease (GD3), a rare lysosomal storage disorder.

Venglustat, which works by reducing the abnormal accumulation of sugar-and-fat molecules in cells and organs, is an investigational glucosylceramide synthase inhibitor (GCSi) that crosses the blood-brain barrier with the goal of targeting some of the neurological aspects of GD3 that currently have no approved therapies. Sanofi has supported the Gaucher disease community for decades as part of an over 40-year commitment to improving care for rare diseases.

The results will be shared this week at the 22nd annual WORLD*Symposium*™ as late-breaking research.

> *"These findings underscore Sanofi's commitment to rare disease research and the promise we aim to deliver for people living with these conditions," said* ***Houman Ashrafian****, Executive Vice President and Head of Research and Development at Sanofi. "What excites us most is the potential to address critical unmet medical needs. A daily pill could make a serious difference for Gaucher patients facing neurological challenges. Most importantly, none of this would be possible without the courage of the patients and families who participate in our studies, and for that we owe them a debt of gratitude."*

In the LEAP2MONO study, GD3 patients receiving venglustat demonstrated statistically significant improvements in neurological symptoms measured by a global test score for two assessments, the Scale for Assessment and Rating of Ataxia (SARA) modified total score and the Repeatable Battery for the Assessment of Neuropsychological Status (RBANS), at week 52 compared with those receiving enzyme replacement therapy (ERT) ($p=0.007$). Venglustat performed as well as ERT on non-neurological outcomes, including changes in spleen volume, liver volume, and hemoglobin levels, three key secondary endpoints of the study.

Venglustat is also being studied for the treatment of Fabry disease, another rare lysosomal storage disorder. Data from the phase 3 PERIDOT study (clinical study identifier: NCT05206773) show that reduction in neuropathic and abdominal pain was observed in both study arms and the primary endpoint was not met. Additional analyses of the data are ongoing

with more information to be shared at a future medical meeting. A second phase 3 study, CARAT (clinical study identifier: NCT05280548), evaluating the effect of venglustat on left cardiac ventricular mass index in men and women with Fabry disease, is ongoing.

Venglustat was well tolerated overall with no new safety signals compared with previous studies. In LEAP2MONO, the most commonly reported adverse events during treatment among patients receiving venglustat (21 individuals) compared with those receiving ERT (22 individuals) were headache (14.3% in the venglustat arm versus 18.2% in the ERT arm), nausea (14.3% versus 4.5%), spleen enlargement (14.3% versus 0), and diarrhea (14.3% versus 0).

Sanofi will pursue global regulatory filings for venglustat in GD3. Venglustat is investigational, and its safety and efficacy have not been evaluated by any regulatory authority.

Sanofi currently markets Fabrazyme, an ERT for Fabry disease, and Cerezyme and Cerdelga for Gaucher disease, an ERT and oral therapy, respectively, in markets around the world. In January 2026, the US approved an expanded label for Cerezyme to include non-central nervous system (CNS) manifestations of GD3, building on its long-standing approval in the US for GD1. This US supplemental label expansion was based exclusively on real-world evidence and leveraged data from the International Collaborative Gaucher Group Gaucher Registry. With the US label expansion, Cerezyme can now be prescribed globally to patients with either GD1 or GD3.

About Gaucher disease

Gaucher disease (GD) is a rare inherited lysosomal storage disease that results from a deficiency of an enzyme called glucocerebrosidase (also known as acid β-glucosidase), leading to the accumulation of molecules called glycosphingolipids (GSLs), particularly in macrophages of the spleen, liver, bone marrow, and lungs. There are three major forms within the clinical spectrum of Gaucher disease: GD1, which is characterized by the lack of (or late) central nervous system (CNS) involvement; GD2, which is the acute neuronopathic form; and GD3, which is the chronic neuronopathic form. In people with GD3, accumulation of GSLs in the CNS can result in neurological manifestations, including ataxia and cognitive deficits, in addition to the systemic manifestations seen in GD1, such as liver and spleen enlargement, anemia, thrombocytopenia, or bone disease. Systemic manifestations of GD3 are treated with ERT, but there are no approved treatments for neurologic manifestations of GD3.

About Fabry disease

Fabry disease is an inherited rare lysosomal storage disease that results from a deficiency of functional alpha-galactosidase A (α-Gal A), leading to a build-up of GSLs, causing progressive cellular accumulation and organ damage in the kidney, cardiovascular and cerebrovascular systems. Neuropathic pain, abdominal pain and other gastrointestinal symptoms are among the first clinical manifestations of Fabry disease and can substantially impact activities of daily living. Clinically, there are two major subtypes: the more severe classic phenotype that presents in childhood with little or no functional α-Gal A enzymatic activity and the non-classic phenotype that presents later in life, typically in adulthood. Non-classic patients have residual α-Gal A activity but progressively accumulate GSLs.

About venglustat

Venglustat is a novel, investigational oral glucosylceramide synthase inhibitor (GCSi), designed to cross the blood brain barrier (i.e., brain-penetrant), that has the potential to slow the progression of certain diseases by inhibiting abnormal GSL accumulation and its physio-pathologic consequences. GSLs are cellular building blocks whose abnormal accumulation is implicated in several rare diseases leading to both cell dysfunction and disease progression. Venglustat was previously granted orphan designation in the EU, the USA and Japan for its

potential treatment of both GD3 and Fabry disease. It also received fast-track designation in the US Food & Drug Administration (FDA) for its potential use in GD3 and Fabry disease.

About the LEAP2MONO study

The LEAP2MONO phase 3 study was a double-blind, double-dummy, active-comparator, two-arm study that evaluated the efficacy and safety of once daily oral venglustat versus intravenous ERT every two weeks in adults and pediatric patients aged 12 and older with GD3. Forty-three patients were randomized [1:1] to receive venglustat and placebo infusion or ERT and placebo tablet. Patients must have been treated with ERT for at least three years and achieved therapeutic goals for systemic disease manifestations. The primary endpoints for the study was change in SARA modified total score and change in RBANS total scale index score for patients receiving venglustat versus those receiving ERT from baseline to week 52. Systemic key secondary endpoints include percent change in spleen volume, liver volume and platelet count and change in hemoglobin levels. Biomarker key secondary endpoints include percent change in cerebrospinal fluid and plasma GL1 and lyso-GL1. The LEAP2MONO study is ongoing and results from its open-label phase will be presented in the future when available.

About the PERIDOT study

The PERIDOT phase 3 study was a double-blind, randomized, placebo-controlled study that evaluated the efficacy and safety of venglustat on neuropathic and abdominal pain in patients aged 16 years and older with Fabry disease. The study randomized 122 patients 1:1 to receive venglustat or placebo. Patients must have been treatment-naïve or untreated for at least six months. The primary endpoint was the percent change from baseline on patient-defined most bothersome symptoms (neuropathic pain in upper extremities, neuropathic pain in lower extremities or abdominal pain), as assessed by the FD-PRO instrument, in those treated with venglustat versus placebo. Secondary endpoints include percent change from baseline in plasma globotriaosylsphingosine (lyso-GL-3), frequency of rescue pain medication use, change from baseline in the percentage of days with diarrhea, percent change from baseline in tiredness component of FD-PRO, and proportion of responders on the patient-defined most bothersome symptoms.

About Sanofi

Sanofi is an R&D driven, AI-powered biopharma company committed to improving people's lives and delivering compelling growth. We apply our deep understanding of the immune system to invent medicines and vaccines that treat and protect millions of people around the world, with an innovative pipeline that could benefit millions more. Our team is guided by one purpose: we chase the miracles of science to improve people's lives; this inspires us to drive progress and deliver positive impact for our people and the communities we serve, by addressing the most urgent healthcare, environmental, and societal challenges of our time.
Sanofi is listed on EURONEXT: SAN and NASDAQ: SNY

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