**Press Release**

# *Sanofi's venglustat accepted for priority review in the US to treat type 3 Gaucher disease*

**Paris, May 28, 2026**. The US Food and Drug Administration (FDA) has granted priority review to the new drug application (NDA) for venglustat, a novel, investigational oral glucosylceramide synthase inhibitor (GCSi), for the treatment of type 3 Gaucher disease (GD3), a rare lysosomal storage disorder.

If approved, venglustat would become the first treatment available in the US to address the progressive neurological manifestations associated with GD3 and expand Sanofi's portfolio of treatment options for patients living with lysosomal storage diseases. The target action date for the FDA decision is November 25, 2026.

Gaucher disease is marked by the abnormal buildup of sugar-and-fat molecules called glycosphingolipids (GSL) in the spleen, liver, bone marrow, and lungs. In patients with GD3, these molecules also accumulate in the central nervous system (CNS) where they drive neuroinflammation, which can result in neurological manifestations, including cognitive deficits and difficulty in coordination and balance (ataxia), in addition to the disease's systemic effects. There are currently no approved targeted treatments that specifically address the neurological symptoms of GD3. By crossing the blood brain barrier, venglustat has the potential to treat neurological manifestations of GD3.

The NDA is supported by positive data from the LEAP2MONO phase 3 study (clinical study identifier: NCT05222906), evaluating the efficacy and safety of venglustat in adults and pediatric patients, with neurological manifestations of GD3, who previously achieved stabilization of systemic manifestations with enzyme replacement therapy (ERT). In results shared at the WORLDSymposium™ earlier this year, venglustat met both of the study's primary endpoints and three out of four key secondary endpoints. In the study, venglustat was well tolerated overall with no new safety signals compared with previous studies. The most commonly reported adverse events 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).

Venglustat previously earned breakthrough therapy designation and fast-track designation from the FDA for its potential in GD3, as well as orphan designation for GD3 in the US, EU, and Japan. Venglustat is also currently under regulatory review for GD3 in the EU. Sanofi will pursue additional global regulatory filings for venglustat in GD3 in 2026.

The safety and efficacy of venglustat for GD3 have not been evaluated by any regulatory authority.

Priority review is given to regulatory applications seeking approval for therapies that have the potential to provide significant improvements in the treatment, diagnosis, or prevention of serious conditions.

### *About LEAP2MONO*

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 were change in scale for assessment and rating of ataxia (SARA) modified total score

and change in repeatable battery for the assessment of neuropsychological status (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 venglustat*

Venglustat is a novel, investigational oral 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 pathophysiology 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 US, and Japan for its potential treatment of both GD3 and Fabry disease. It also received fast-track designation by the FDA for its potential use in GD3 and Fabry disease.

### *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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***Sanofi forward-looking statements***

This press release contains forward-looking statements within the meaning of applicable securities laws, including 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 events and economic performance. Words such as "expect," "anticipate," "believe," "intend," "estimate," "plan," "can," "contemplate," "could," "is designed to," "may," "might," "potential," "objective," "attempt," "target," "project," "strategy," "strive," "desire," "predict," "forecast," "ambition," "guideline," "seek," "should," "will," "goal," or the negative of these, and similar expressions are intended to identify forward-looking statements. 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 U.S Food and Drug Administration or the European Medicines Agency, 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 fact that product candidates if approved may not be commercially successful; unexpected regulatory actions or delays, or government regulation generally; authorities' decisions regarding whether and when to approve a product candidate; political pressure in the United States to mandate lower drug prices including "most favored nation" pricing for State Medicaid programs; the future approval and commercial success of therapeutic alternatives; Sanofi's ability to benefit from external growth opportunities, to complete related transactions and/or obtain regulatory clearances, including future clinical data and analysis of existing clinical data relating to the product, including post marketing, unexpected safety, quality or manufacturing issues, competition in general; 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 and market conditions, cost containment initiatives and subsequent changes thereto, and the impact that global crises may have on us, our customers, suppliers, vendors, and other business partners, and

the financial condition of any one of them, as well as on our employees and on the global economy as a whole. The risks and uncertainties also include the uncertainties discussed or identified in the public filings with the SEC and the French Markets Authority (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, 2025, or contained in our periodic reports on Form 6-K. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements. In light of these risks, uncertainties, and assumptions, you should not place undue reliance on any forward-looking statements contained herein.