Press Release

Sanofi's rilzabrutinib designated breakthrough therapy in the US and orphan drug in Japan for the treatment of warm autoimmune hemolytic anemia

- Rilzabrutinib is the first and only investigational BTKi for warm autoimmune hemolytic anemia to be designated Breakthrough Therapy by the FDA
- Rilzabrutinib helps address complex immune-system dysregulation through multi-immune modulation
- Rilzabrutinib holds global regulatory designations across multiple rare diseases, underscoring its broad therapeutic potential

Paris, February 9, 2026. The US Food and Drug Administration (FDA) has granted a designation as breakthrough therapy to Wayrilz (rilzabrutinib), a novel oral, reversible Bruton's tyrosine kinase (BTK) inhibitor, for the treatment of patients with warm autoimmune hemolytic anemia (wAIHA), a rare autoimmune disorder marked by the destruction of red blood cells. The Japanese Ministry of Health, Labour and Welfare has also provided rilzabrutinib an orphan designation for the same condition.

Both designations are based on clinical data from the ongoing LUMINA 2 phase 2b study (clinical study identifier: NCT05002777) assessing the efficacy and safety of rilzabrutinib for patients with wAIHA. In addition, the new LUMINA 3 phase 3 study (clinical study identifier: NCT07086976), is assessing rilzabrutinib compared with placebo in patients with wAIHA. There is currently no approved treatment that specifically targets the underlying cause of this rare autoimmune condition, which can lead to anemia, fatigue, and serious organ damage.

An FDA breakthrough therapy designation is designed to expedite the development and review of medicines in the US intended to treat serious or life-threatening conditions and where preliminary clinical evidence indicates the therapy may demonstrate substantial improvement over available treatment options. Orphan designation in Japan is granted to medicines intended to address rare diseases with high unmet medical need.

> *"These recognitions highlight the critical unmet need that persists for people living with wAIHA,"* said **Karin Knobe**, Global Head of Development, Rare Diseases. *"Furthermore, receiving such designations reinforces our commitment to advancing innovative medicines for rare diseases that currently have limited or no approved treatment options."*

Rilzabrutinib is approved in the US, the EU, and the United Arab Emirates (UAE) under the brand name Wayrilz for the treatment of adults with immune thrombocytopenia (ITP) and is currently under regulatory review for ITP in Japan. Other than the approved ITP indications in the US, EU, and UAE, these uses of rilzabrutinib are investigational and have not been evaluated by any regulatory authority.

The FDA previously granted rilzabrutinib orphan drug designation for autoimmune hemolytic anemia, as well as two other rare diseases, IgG4-related disease (IgG4-RD) and sickle cell disease (SCD). Rilzabrutinib also received FDA fast track designation for ITP and IgG4-RD and EU orphan designation in ITP, autoimmune hemolytic anemia, and IgG4-RD.

About wAIHA

wAIHA is a rare, potentially life-threatening, autoimmune disorder rooted in complex immune system dysregulation. It represents more than half of autoimmune hemolytic anemia cases. In

wAIHA, autoantibodies lead to the premature destruction of the body's own red blood cells (hemolysis), sometimes faster than the bone marrow can replace them. In the US and EU, autoimmune hemolytic anemia is estimated to affect four to 24 people out of 100,000, while in Japan it is rarer, affecting three to 10 people per million. People living with wAIHA may experience debilitating fatigue, dizziness, palpitations, and shortness of breath, and may face complications such as thromboembolism.

About rilzabrutinib

Rilzabrutinib, Wayrilz where approved, is a novel, oral, reversible covalent BTK inhibitor that has the potential to be an effective new medicine for several rare immune-mediated or inflammatory diseases by working to restore immune balance via multi-immune modulation. BTK, expressed in B cells, macrophages, and other innate immune cells, plays a critical role in multiple immune-mediated disease processes and inflammatory pathways. With the application of the TAILORED COVALENCY® technology, rilzabrutinib can selectively inhibit the BTK target. Wayrilz is now approved for the treatment of immune thrombocytopenia (ITP) in the US, the EU, and the UAE. Regulatory review for use in ITP is currently ongoing in Japan.

In addition to ITP and wAIHA, rilzabrutinib is being studied across a variety of rare diseases, including IgG4-RD and SCD. These additional indications are currently under investigation and have not been approved by regulatory authorities.

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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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 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, 2024. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.