

Sanofi's rilzabrutinib earns orphan drug designation in Japan for IgG4-related disease

- Designation based on positive data from a phase 2 study of rilzabrutinib in IgG4-RD
- Third global orphan drug designation for rilzabrutinib in IgG4-RD, underpinning Sanofi's commitment to rare immune-mediated diseases

Paris, March 2, 2026 – The Ministry of Health, Labour and Welfare (MHLW) in Japan has granted orphan drug designation to rilzabrutinib, a novel, oral, reversible covalent Bruton's tyrosine kinase (BTK) inhibitor, for IgG4-related disease (IgG4-RD). There is still unmet medical need and limited treatment options in Japan for IgG4-RD, a rare, progressive, immune-mediated chronic condition in which the immune system attacks various tissues and organs leading to serious damage. The MHLW grants orphan designation to medicines that address rare diseases or conditions with unmet medical needs.

Rilzabrutinib for the treatment of IgG4-RD was evaluated in a phase 2 study (clinical study identifier: [NCT04520451](#)) and results were [presented](#) at the European Alliance of Associations for Rheumatology 2025 congress. In IgG4-RD patients, treatment with rilzabrutinib for 52 weeks led to reduction in disease flares and other disease markers and minimized the need for treatment with glucocorticoids. The safety profile of rilzabrutinib in the study was consistent with previous studies in other indications, with no new safety signals observed. Treatment-emergent adverse events reported by >10% of patients include diarrhea, COVID-19, dizziness, dry mouth and nausea. Currently, rilzabrutinib in IgG4-RD is being evaluated in the RILIEF phase 3 study (clinical study identifier: [NCT07190196](#)).

Rilzabrutinib is being studied across multiple rare immune-mediated diseases. In 2025, it received approval for immune thrombocytopenia (ITP) in the US, the EU, and the UAE. Additionally, rilzabrutinib is currently under regulatory review for ITP in Japan. Rilzabrutinib has received several expedited designations from global regulatory authorities for ITP, IgG4-RD, warm autoimmune hemolytic anemia (wAIHA), and sickle cell disease (SCD). 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.

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. Rilzabrutinib 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.

About IgG4-RD

IgG4-RD is a progressive, relapsing, chronic immune-mediated rare disease, which can manifest in almost every organ and can lead to organ damage and irreversible dysfunction with a sometimes fatal outcome. People with IgG4-RD experience flare-ups of the condition characterized by periods of exacerbated symptoms. Due to its rarity and challenges with diagnosis, the global prevalence of IgG4-RD is unknown.

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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