Press Release

Rilzabrutinib granted orphan drug designation in the US for sickle cell disease

- Fourth orphan drug designation for rilzabrutinib in rare diseases
- Under regulatory review in the US, the EU, and China in immune thrombocytopenia

Paris, June 3, 2025. The US Food and Drug Administration (FDA) has granted orphan drug designation to rilzabrutinib, a novel, advanced, oral, reversible Bruton's tyrosine kinase (BTK) inhibitor that works via multi-immune modulation, to target a reduction in vaso-occlusive crises, which may occur via inflammation, in sickle cell disease. The FDA grants orphan drug designation to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the US.

> ***Karin Knobe, MD, PhD***
> Global Head of Development, Rare Diseases
> *"Receiving our fourth orphan drug designation for rilzabrutinib reinforces our continued dedication to developing medicines to address the unmet medical needs of people living with rare diseases. People with sickle cell disease often live with severe episodes of pain from vaso-occlusive crises and other complications that can significantly impact both quality of life and life expectancy. There remains a need for novel treatment approaches to address these experiences by modulating the immune system responses that can contribute to sickle cell disease pathogenesis."*

In addition to sickle cell disease, rilzabrutinib has received orphan drug designation for immune thrombocytopenia (ITP) in the US, the EU, and Japan, for warm autoimmune hemolytic anemia (wAIHA) in the US and the EU, and for IgG4-related disease (IgG4-RD) in the US.

The safety and efficacy of rilzabrutinib have not been determined by any regulatory authority. Rilzabrutinib is currently under regulatory review in the US, the EU, and in China for its potential use in ITP. The target action date for the FDA regulatory decision for ITP, which was granted fast track designation, is August 29, 2025.

Sickle cell disease supporting data

Preclinical data on sickle cell disease was presented at ASH 2024 showing that rilzabrutinib helped reduce vaso-occlusion –blockage of blood vessels – and inflammation in transgenic mice with sickle cell disease.

About rilzabrutinib

Rilzabrutinib is a novel, advanced, oral, reversible Bruton's tyrosine kinase (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 Sanofi's TAILORED COVALENCY® technology, rilzabrutinib can selectively inhibit the BTK target while potentially reducing the risk of off-target side effects.

About sickle cell disease

Sickle cell disease is a group of rare, genetic blood disorders in which red blood cells are misshapen, typically in a sickle or crescent shape, causing them to get stuck in small blood vessels, blocking blood flow. This can lead to episodes of severe pain as well as other health complications including infections, stroke, lung, eye, and kidney disease. Sickle cell disease affects more than 100,000 people in the US, approximately 90% of whom are African American and 3-9% of whom are Hispanic or Latino. Approximately 1 in 365 babies of African

American descent born in the US have sickle cell disease, while an estimated 1 in 13 are carriers of the disease. People in the US with sickle cell disease have an estimated life expectancy that is 20 years shorter than average.

About Sanofi

Sanofi is an R&D driven, AI-powered biopharma company committed to improving people's lives and creating 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.

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