Press Release

Sanofi's Wayrilz approved in US as first BTK inhibitor for immune thrombocytopenia

- Novel treatment targets BTK through multi-immune modulation to help address root causes of ITP
- Approval based on LUNA 3 phase 3 study that demonstrated rapid and durable platelet response and improvements in other ITP symptoms
- ITP is a disease of complex immune dysregulation leading to low platelet counts, bleeding, and reduced quality of life; more than 25,000 US adults could benefit from advanced treatment

Paris, August 29, 2025 – The US Food and Drug Administration (FDA) has approved Wayrilz (rilzabrutinib) for adults with persistent or chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment. The approval was based on the pivotal LUNA 3 phase 3 study, in which Wayrilz met the primary and secondary endpoints, showing a positive impact on sustained platelet counts and other ITP symptoms.

> *"The burden of immune thrombocytopenia can be both physical and emotional with significant overlooked symptoms that can impact various aspects of daily living," said* ***Caroline Kruse****, President and CEO at the Platelet Disorder Support Association. "We are pleased to have a new treatment option that can help ease the ongoing strain of managing the disease for patients and their families."*

As a novel oral, reversible, Bruton's tyrosine kinase (BTK) inhibitor, Wayrilz can help address the root causes of ITP through multi-immune modulation, targeting different pathways across the immune system.

> "*With its differentiated mechanism of action, Wayrilz has the potential to become a treatment of choice for immune thrombocytopenia patients who have not responded to a prior therapy," said* ***Brian Foard****,* Executive Vice President, Head of Specialty Care at Sanofi. *"Its multi-immune modulation approach shows promise in addressing the key drivers of immune thrombocytopenia, which aligns with Sanofi's commitment to adapting and evolving therapeutic solutions to help tackle ongoing unmet patient needs. This approval underscores Sanofi's expertise and ambitions at the junction of rare and immunological disease."*

The LUNA 3 phase 3 study, presented at the 66th American Society of Hematology Annual Meeting and Exposition, evaluated the efficacy and safety of Wayrilz compared to placebo in adults (n=202) with persistent or chronic ITP. Patients who achieved platelet count response at 12 weeks were eligible to continue the full 24-week double-blind period (64% of patients in the Wayrilz arm and 32% of patients in the placebo arm). Patients receiving Wayrilz experienced the following compared to patients receiving placebo:

- Statistically significant durable platelet response at week 25 (23% of patients in Wayrilz arm vs. 0% in placebo arm; $p<0.0001$)
- Faster time to first platelet response (36 days in Wayrilz arm vs. not reached in placebo arm; $p<0.0001$)
- Longer duration of platelet response (least square mean of 7 weeks in Wayrilz arm vs. 0.7 weeks in placebo arm)

Patients receiving Wayrilz reported an overall 10.6-point improvement across nine health-related quality of life measures compared to a 2.3-point increase in the placebo arm, based on The Immune Thrombocytopenia Patient Assessment Questionnaire, a clinical tool designed to measure ITP symptoms. The results of this analysis are descriptive and were not powered for statistical significance.

The most common adverse reactions (incidence ≥10%) are diarrhea, nausea, headache, abdominal pain, and COVID-19.

> *"Traditionally, immune thrombocytopenia management has focused on restoring platelet counts and reducing bleeding risk, which for some patients may result in suboptimal responses, persistent symptoms, or unacceptable treatment complications," said* ***David Kuter, MD****, Director of Clinical Hematology at Massachusetts General Hospital and Professor of Medicine at Harvard Medical School, study author. "Through multi-immune modulation, Wayrilz can offer a new option for patients, including those who fail steroids or do not respond to existing treatment."*

Wayrilz was approved in the United Arab Emirates for adult patients with persistent or chronic ITP who have had an insufficient response or intolerance to a previous treatment in June 2025. Wayrilz is currently under regulatory review for ITP in the EU and China.

It received Fast Track and Orphan Drug Designations (ODD) from the FDA for ITP, with similar orphan designations in Japan and the EU. Most recently, the FDA granted Wayrilz ODD for three additional rare diseases, including warm autoimmune hemolytic anemia (wAIHA), IgG4-related disease (IgG4-RD), and sickle cell disease (SCD). Wayrilz also received FDA Fast Track Designation and European Medicines Agency orphan designation in IgG4-RD.

Wayrilz patients will have access to Sanofi's HemAssist patient support program that offers assistance for all treatments within Sanofi's rare blood disorder portfolio. HemAssist aims to help patients and their caregivers with the support they need throughout their treatment journey, including navigation of access and insurance coverage, determining eligibility for financial assistance programs, and providing educational resources. Patient costs for treatment will vary based on insurance and patients without insurance coverage can work with HemAssist to identify potential options by visiting https://www.sanofihemassist.com or by calling 1-833-723-5463.

About the LUNA 3 study

LUNA 3 (clinical study identifier: NCT04562766) was a randomized, multicenter, phase 3 study evaluating the efficacy and safety of Wayrilz vs. placebo in adult and adolescent patients with persistent or chronic ITP. Patients received either oral Wayrilz 400 mg twice a day or placebo through a 12- to 24-week double-blind treatment period, followed by a 28-week open-label treatment period, and then a 4-week safety follow-up or long-term extension period. The adolescent part of the study is ongoing. The primary endpoint is durable platelet response, defined as the proportion of participants able to achieve platelet counts at or above 50,000/µL for more than two-thirds of at least 8 non-missing weekly scheduled platelet measurements during the last 12 weeks of the 24-week blinded treatment period in the absence of rescue therapy. Secondary endpoints included time to platelet response (platelet count ≥50 x 10^9/L or between 30x10^9/L and <50x10^9/L and at least doubled from baseline in absence of rescue therapy), number of weeks maintaining a specific platelet response (i.e., doubled or within range), rescue therapy use, physical fatigue score, and bleeding score.

About Wayrilz

Wayrilz (rilzabrutinib) is the first BTK inhibitor for ITP that helps address the root cause of disease through multi-immune modulation. This innovative therapy is approved in the US for adults with persistent or chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment. Additionally, Wayrilz is approved in the United Arab Emirates for adult patients with persistent or chronic ITP who have had an insufficient response or intolerance to a previous treatment. 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, Wayrilz can selectively inhibit the BTK target.

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

About ITP

ITP is a disease of complex immune dysregulation that causes low platelet counts (<100,000/µL), resulting in a variety of bleeding symptoms and thromboembolism risk. Beyond bruising and bleeding, which can include potentially life-threatening episodes like intracranial hemorrhage, people living with ITP may experience reduced quality of life, including physical fatigue and cognitive impairment.

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

Media Relations

Sandrine Guendoul | +33 6 25 09 14 25 | sandrine.guendoul@sanofi.com
Evan Berland | +1 215 432 0234 | evan.berland@sanofi.com
Léo Le Bourhis | +33 6 75 06 43 81 | leo.lebourhis@sanofi.com
Victor Rouault | +33 6 70 93 71 40 | victor.rouault@sanofi.com
Timothy Gilbert | +1 516 521 2929 | timothy.gilbert@sanofi.com
Léa Ubaldi | +33 6 30 19 66 46 | lea.ubaldi@sanofi.com

Investor Relations

Thomas Kudsk Larsen |+44 7545 513 693 | thomas.larsen@sanofi.com
Alizé Kaisserian | +33 6 47 04 12 11 | alize.kaisserian@sanofi.com
Felix Lauscher | +1 908 612 7239 | felix.lauscher@sanofi.com
Keita Browne | +1 781 249 1766 | keita.browne@sanofi.com
Nathalie Pham | +33 7 85 93 30 17 | nathalie.pham@sanofi.com
Tarik Elgoutni | +1 617 710 3587 | tarik.elgoutni@sanofi.com
Thibaud Châtelet | +33 6 80 80 89 90 | thibaud.chatelet@sanofi.com
Yun Li | +33 6 84 00 90 72 | yun.li@sanofi.com

Sanofi forward-looking statements

This press release contains forward-looking statements as defined in 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 regarding the marketing and other potential of the product, or regarding potential future revenues from the product. Forward-looking statements are generally identified by the words "expects", "anticipates", "believes", "intends", "estimates", "plans", and similar expressions. 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, unexpected regulatory actions or delays, or government regulation generally, that could affect the availability or commercial potential of the product, the fact that product may not be commercially successful, the uncertainties inherent in research and development, 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 future litigation and the ultimate outcome of such litigation, and volatile economic and market conditions, 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 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.