

## *Positive Phase 1/2 study results of rilzabrutinib in people with immune thrombocytopenia published in The New England Journal of Medicine*

- Results published today in the New England Journal of Medicine showed that treatment with rilzabrutinib resulted in a rapid and durable increase in platelet count in patients with heavily pretreated immune thrombocytopenia (ITP)
- Data support an acceptable safety profile
- Rilzabrutinib is an investigational oral Bruton tyrosine kinase inhibitor (BTKi) for the treatment of ITP, a rare acquired autoimmune disorder in which platelets are destroyed or damaged and for which there are limited treatment options

**PARIS – April 14, 2022** - Positive results from the Phase 1/2 dose-finding study evaluating the safety, pharmacokinetics and clinical activity of rilzabrutinib, an investigational oral Bruton's tyrosine kinase (BTK) inhibitor, in adults with heavily pre-treated immune thrombocytopenia (ITP) were published in the *New England Journal of Medicine*. Results demonstrate treatment with rilzabrutinib led to a rapid and durable increase in platelet count and support an acceptable safety profile. Sanofi is investigating the safety and efficacy of twice daily rilzabrutinib (400 mg) for adults and adolescents with chronic ITP in the ongoing Phase 3 clinical study LUNA 3, initiated in April 2021.

***David Kuter, M.D.***

Director of clinical hematology at Massachusetts General Hospital and professor of medicine at Harvard Medical School, lead author of the study

*“Currently, there are no standard treatment recommendations for ITP patients with multiple relapses. Despite advances in treatment options over the years, some patients remain refractory to existing therapies and durable remission remains elusive. The Bruton's tyrosine kinase is a critical signaling molecule in the immune system that is involved in certain immune-mediated diseases, and our research suggests that targeting BTK may represent a promising approach to addressing the underlying cause of ITP.”*

ITP is an acquired autoimmune blood disorder characterized by low platelet count (thrombocytopenia) resulting from immune-mediated platelet destruction and impairment of platelet production. A decrease in platelet counts – whether temporary or persistent – can predispose a person to a higher risk of bleeding, hospitalization, fatigue, impaired quality of life, and even death. The incidence of ITP increases with age and is more common over the age of 60.

***Dietmar Berger, M.D., Ph.D.***

Global Head of Clinical Development and Chief Medical Officer, Sanofi

*“We are pleased to share these encouraging early clinical results through this publication. These findings demonstrate a clinically meaningful response in difficult-to-treat ITP patients who received a median of four prior ITP therapies. Moreover, the overall study population, which also included less refractory patients, showed a numerically higher response. Rilzabrutinib could become a first-in-class BTK inhibitor therapy with the potential to increase platelet counts quickly and durably for people with ITP.”*

Rilzabrutinib was granted Fast Track Designation by the U.S. Food and Drug Administration (FDA) for treatment of ITP in November of 2020 and was previously granted orphan drug designation. Rilzabrutinib is being investigated in multiple clinical trials across a range of diseases including immunological and inflammatory diseases.

## Phase 1/2 Study Results

The global Phase 1/2 adaptive, open-label, dose-finding study evaluated rilzabrutinib in 60 people with ITP with a median age of 50 years (range, 19-74). Patients had received a median of four different ITP therapies previously. Initial doses could be 200 mg once daily, 400 mg once daily, 300 mg twice daily (600 mg/day), or 400 mg twice daily (800 mg/day). The median platelet counts at the start of the study were  $15 \times 10^9/L$ , indicating a very low platelet count and high risk of bleeding. The primary endpoint measured the number of participants who achieved at least two consecutive platelet counts of  $\geq 50 \times 10^9/L$  and an overall platelet count increase of  $\geq 20 \times 10^9/L$  from the start of treatment without requiring rescue medication.

Study results showed:

- Overall, 24 of 60 people enrolled in the study at any dose achieved the primary endpoint. Of the 45 people who initiated rilzabrutinib at 400 mg twice daily, 18 met the primary endpoint.
- Median time to first platelet count of at least  $50 \times 10^9/L$  was rapid at 11.5 days, which was maintained in patients with primary platelet response for a mean of 65% of weeks during the 24-week treatment period.
- 52% of participants experienced at least one treatment related adverse event, all of which were grade 1 or 2; the most common adverse events were diarrhea (32%), nausea (30%), and fatigue (10%).
- There were no grade 3 or higher treatment-related adverse events or serious adverse events.

## Rilzabrutinib Clinical Program

The safety and efficacy of rilzabrutinib in ITP are being evaluated in the ongoing randomized, double-blind, Phase 3 LUNA 3 study in adults and adolescents (aged  $\geq 12$  years) with persistent/chronic ITP. In addition, phase 2 studies are ongoing to evaluate rilzabrutinib as a potential therapy for the autoimmune condition IgG4 disease and immunological diseases, including asthma, atopic dermatitis, chronic spontaneous urticaria and warm autoimmune hemolytic anemia.

## About Rilzabrutinib

Rilzabrutinib is an oral Bruton's tyrosine kinase inhibitor incorporating Sanofi's TAILORED COVALENCY® technology being investigated for the treatment of immune-mediated diseases, including ITP. BTK is an intracellular signaling molecule involved in innate and adaptive immune responses related to certain immune-mediated diseases. By inhibiting BTK, rilzabrutinib has the potential to target the underlying disease pathogenesis.

Rilzabrutinib is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority.

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### About Sanofi

We are an innovative global healthcare company, driven by one purpose: we chase the miracles of science to improve people's lives. Our team, across some 100 countries, is dedicated to transforming the practice of medicine by working to turn the impossible into the possible. We provide potentially life-changing treatment options and life-saving vaccine protection to millions of people globally, while putting sustainability and social responsibility at the center of our ambitions.

Sanofi is listed on Euronext: SAN and NASDAQ: SNY

### Media Relations

Sally Bain | + 1 617 834 6026 | [sally.bain@sanofi.com](mailto:sally.bain@sanofi.com)

### Investor Relations

Eva Schaefer-Jansen | + 33 7 86 80 56 39 | [eva.schaefer-jansen@sanofi.com](mailto:eva.schaefer-jansen@sanofi.com)

Arnaud Delépine | + 33 6 73 69 36 93 | [arnaud.delepine@sanofi.com](mailto:arnaud.delepine@sanofi.com)

**Corentine Driancourt** | + 33 6 40 56 92 21 | [corentine.driancourt@sanofi.com](mailto:corentine.driancourt@sanofi.com)  
**Felix Lauscher** | + 1 908 612 7239 | [felix.lauscher@sanofi.com](mailto:felix.lauscher@sanofi.com)  
**Priya Nanduri** | +1 908 981 5560 | [priya.nanduri@sanofi.com](mailto:priya.nanduri@sanofi.com)  
**Nathalie Pham** | + 33 7 85 93 30 17 | [nathalie.pham@sanofi.com](mailto:nathalie.pham@sanofi.com)

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