

## Rilzabrutinib granted FDA Fast Track Designation for treatment of immune thrombocytopenia

- \* Phase 3 trial initiated to evaluate rilzabrutinib, the potential first BTK inhibitor (Bruton's tyrosine kinase inhibitor) for the treatment of immune thrombocytopenia
- \* Rilzabrutinib previously granted FDA orphan drug designation

**PARIS – November 18, 2020** – The U.S. Food and Drug Administration (FDA) has granted Fast Track Designation (FTD) to the oral investigational Bruton's tyrosine kinase (BTK) inhibitor, rilzabrutinib, which has the potential to be the first BTK inhibitor for the treatment of immune thrombocytopenia (ITP). In addition, following positive Phase 1/2 study results, a Phase 3 study evaluating rilzabrutinib for ITP has been initiated. Rilzabrutinib received orphan drug designation from the FDA for the treatment of ITP in October 2018.

*"By awarding Fast Track Designation to rilzabrutinib, an investigational candidate for the treatment of ITP, the FDA has recognized rilzabrutinib's potential to meaningfully improve outcomes for patients with this debilitating disease. This is an excellent acknowledgement as we initiate our Phase 3 study,"* said Dolca Thomas, Chief Medical Officer of Principia, a Sanofi company. *"FTD is designed to facilitate the development and expedite the review of investigational treatments that demonstrate the potential to address unmet medical needs in serious or life-threatening conditions,"*

### About Fast Track Designation

FTD is an FDA process designed to facilitate the development, and expedite the review of, medicines to treat serious conditions and fill unmet medical need. The FDA created this process to help deliver important new drugs to patients earlier, and it covers a broad range of serious illnesses. Fast Track designation can lead to an Accelerated Approval and Priority Review if certain criteria are met.

### About Immune thrombocytopenia

ITP is characterized by immune-mediated platelet destruction and impairment of platelet production, which leads to downstream thrombocytopenia, a predisposition to bleeding, and adverse impact on patient quality of life. There remains an unmet medical need in ITP to achieve rapid and durable remission rates for patients who have relapsed or are refractory to corticosteroids.

## About Rilzabrutinib

Rilzabrutinib is an oral, reversible covalent, Bruton's tyrosine kinase (BTK) inhibitor being investigated for the treatment of immune mediated diseases. BTK is involved in innate and adaptive immune responses and is a signalling molecule in immune mediated diseases. Rilzabrutinib data demonstrate an ability to block inflammatory immune cells, eliminate autoantibody destructive signalling, and prevent new autoantibody production without depleting B cells. Rilzabrutinib has the potential to target the underlying disease pathogenesis and has not been shown to alter platelet aggregation. The clinical significance of these mechanisms is currently under investigation and its safety and efficacy have not been reviewed by any regulatory authority.

**Editor's Note:** Rilzabrutinib is being investigated in a Phase 3 trial for pemphigus, an immune mediated disease characterized by blisters in mucous membranes and skin. Additionally, a Phase 2 study in the autoimmune condition IgG4 Disease has also been initiated.

### About Sanofi

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With more than 100,000 people in 100 countries, Sanofi is transforming scientific innovation into healthcare solutions around the globe.

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#### Sanofi Forward-Looking Statements

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