

## New data to be featured at EHA 2021 Congress highlight Sanofi's ongoing commitment to rare blood disorders

- \* New data and analyses to be presented from the pivotal Phase 3 CADENZA and CARDINAL studies of sutimlimab, a first-in-class investigational C1s inhibitor that has the potential to be the first approved treatment for hemolysis in adults with cold agglutinin disease (CAD), a serious and chronic autoimmune hemolytic anemia
- \* Additional data to be shared on rilzabrutinib, an investigational, peripheral Bruton's tyrosine kinase (BTK) inhibitor with Tailored Covalency® technology intended to treat immune thrombocytopenic purpura (ITP)

**May 12, 2021**

New pivotal data and clinical analyses from Sanofi's portfolio of investigational therapies for immune-mediated rare blood disorders will be featured at the [26<sup>th</sup> Annual European Hematology Association \(EHA\) Virtual Congress](#), June 9-17, 2021.

*"The data being presented at EHA 2021 demonstrate Sanofi's commitment to providing first-in-class and potentially transformative treatments for people with immune-mediated rare blood disorders, where significant unmet needs persist," said Karin Knobe, Head of Development, Rare and Rare Blood Disorders, Sanofi. "We are excited to share pivotal data from the placebo-controlled CADENZA Phase 3 study of sutimlimab in people with CAD with no recent history of transfusion as well as interim data from our rilzabrutinib Phase 1/2 study in patients with ITP."*

### Working to break barriers in the treatment of cold agglutinin disease

Cold agglutinin disease (CAD) is a rare, chronic autoimmune hemolytic anemia that causes the body's immune system to mistakenly attack healthy red blood cells and cause their destruction (hemolysis) via activation of the classical complement pathway. CAD patients may experience chronic anemia, profound fatigue, acute hemolytic crisis, and other potential complications, including an increased risk of thromboembolic events and early death.<sup>1,2,3</sup> CAD impacts the lives of an estimated 12,000 people in the U.S., Europe, and Japan.<sup>4</sup> Currently there are no approved therapies for CAD.

Sutimlimab is an investigational, potential first-in-class monoclonal antibody designed to selectively target C1-activated hemolysis in CAD.

*New data from pivotal Phase 3 CADENZA and CARDINAL studies*

- **Abstract #S291:** C1s-Targeted Inhibition of Classical Complement Pathway by Sutimlimab in Cold Agglutinin Disease (CAD): Efficacy and Safety Results from the Randomized, Placebo (PBO)-Controlled Phase 3 CADENZA Study. Oral Presentation.
- **Abstract #S290:** Sutimlimab, a Targeted Complement C1s Inhibitor, Improves Quality of Life (QOL) in Patients with Cold Agglutinin Disease (CAD): Results from the Randomized, Placebo-Controlled Phase 3 CADENZA Study. Oral Presentation.
- **Abstract #S312:** Sustained Improvements in Patient-Reported Outcomes with Sutimlimab in Patients with Cold Agglutinin Disease: 1-Year Follow-Up Interim Results from the CARDINAL Study. Oral Presentation.
- **Abstract #EP1179:** Clinically Important Change in FACIT-Fatigue Score for Patients with Cold Agglutinin Disease: An Analysis Using the Phase 3 CARDINAL and CADENZA Studies. Poster Presentation.
- **Abstract #EP689:** Inhibition of Complement C1s with Sutimlimab in Patients with Cold Agglutinin Disease (CAD): 1-Year Interim Results of the Phase 3 CARDINAL Study Long-term Follow-up (adaptation). Poster Presentation.

*Data on the potential impact of sutimlimab on QOL and fatigue in people with CAD*

- **Abstract #EP709:** Inflammation and Fatigue in Patients with Cold Agglutinin Disease (CAD): Analysis from the Phase 3 CARDINAL Study (adaptation). Poster Presentation.

*New analyses on disease burden and healthcare resource utilization by people living with CAD*

- **Abstract #EP1180:** Increased Antidepressant Use Among Newly Diagnosed Patients with Cold Agglutinin Disease Compared with Other Patients in a Large US Healthcare System. Poster Presentation.
- **Abstract #EP1193:** Healthcare Resource Utilization Among Patients with Cold Agglutinin Disease in Denmark (adaptation). Poster Presentation.

## **Aiming to address unmet needs in immune thrombocytopenic purpura**

Immune thrombocytopenic purpura (ITP) is an acquired autoimmune blood disorder characterized by immune-mediated platelet destruction and impairment of platelet production, which leads to thrombocytopenia, a predisposition to bleeding, and altered quality of life for patients. There remains a need in relapsed/refractory ITP for patients failing to maintain their platelet counts with the current therapies.

Rilzabrutinib is an investigational peripheral Bruton's tyrosine kinase (BTK) inhibitor with Tailored Covalency technology intended to treat ITP.

- **Abstract #S299:** Phase I/II Updated Safety and Efficacy Results of Oral Bruton Tyrosine Kinase (BTK) Inhibitor Rilzabrutinib in Relapsed/Refractory Immune Thrombocytopenia (ITP). Oral Presentation.

- **Abstract #PB1733:** Phase III Multicenter, Double-Blind, Randomized, Placebo-Controlled Study of Rilzabrutinib, Oral BTK Inhibitor, in Adults and Adolescents with Persistent or Chronic Immune Thrombocytopenia. Abstract-only.

#### Additional presentation

- **Abstract #EP1145:** Epidemiology, Treatment Patterns, and Clinical Outcomes Among Patients with Acquired Thrombotic Thrombocytopenic Purpura (aTTP) in the United States: an Electronic Health Records Analysis

#### Editor's Note:

**About sutimlimab:** Sutimlimab is an investigational, humanized monoclonal antibody that is designed to selectively target and inhibit C1s in the classical complement pathway, which is part of the innate immune system. By blocking C1s, sutimlimab inhibits the activation of the classical complement pathway with the goal of halting C1-activated hemolysis in CAD to prevent the abnormal destruction of healthy red blood cells. By selectively inhibiting the classical pathway upstream at C1s, sutimlimab does not inhibit the lectin and alternative complement pathways.

Sutimlimab has been granted Breakthrough Therapy by the U.S. Food and Drug Administration (FDA) and Orphan Drug status by the FDA, European Medicines Agency and the Pharmaceuticals and Medical Devices Agency in Japan. Sutimlimab is currently under clinical investigation and has not been approved by any regulatory authority.

**About rilzabrutinib:** Rilzabrutinib is an oral, peripheral Bruton's tyrosine kinase inhibitor with Tailored Covalency technology being investigated for the treatment of immune-mediated diseases, including immune thrombocytopenic purpura. BTK is involved in innate and adaptive immune responses and is a signalling molecule in immune mediated diseases. Rilzabrutinib has the potential to target the underlying disease pathogenesis and has not been shown to alter platelet aggregation<sup>5</sup>. The clinical significance of these mechanisms is currently under investigation and have not been approved by any regulatory authority. A Phase 3 clinical trial to evaluate rilzabrutinib for the treatment of ITP is currently underway with the first patient dosed in April 2021. Rilzabrutinib has been granted orphan drug designation and fast track designation by the U.S. FDA for ITP.

Rilzabrutinib is also being investigated in a Phase 3 trial for pemphigus, an immune-mediated disease characterized by blisters in mucous membranes and skin as well as a Phase 2 study in the autoimmune condition IgG4 disease. Three additional Phase 2 studies in immunological diseases are planned to start in 2021.

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\*Tailored Covalency is a registered trademark of Principia Biopharma Inc., a Sanofi company

1. Broome C, et al. Increased risk of thrombotic events in cold agglutinin disease: A 10-year retrospective analysis. *Res Pract Thromb Haemost.* 2020;00:1–8.
2. Quentin A. Hill, Rajeshwari Punekar, Jaime Morales Arias, Catherine M Broome, Jun Su; Mortality Among Patients with Cold Agglutinin Disease in the United States: An Electronic Health Record (EHR)-Based Analysis. *Blood* 2019; 134 (Supplement\_1): 4790.

3. Lauren C. Bylsma, Anne Gulbech Ording, Adam Rosenthal, Buket Öztürk, Jon P. Fryzek, Jaime Morales Arias, Alexander Röth, Sigbjørn Berentsen; Occurrence, thromboembolic risk, and mortality in Danish patients with cold agglutinin disease. *Blood Adv* 2019; 3 (20): 2980–2985.
4. Berentsen S, et al. *Haematologica*. 2006;91(4):460-466
5. Langrish CL et al - Preclinical Efficacy and Anti-Inflammatory Mechanisms of Action of the Bruton Tyrosine Kinase Inhibitor Rilzabrutinib for Immune-Mediated Disease - *J Immunol* published online 5 March 2021

## About Sanofi

Sanofi is dedicated to supporting people through their health challenges. We are a global biopharmaceutical company focused on human health. We prevent illness with vaccines, provide innovative treatments to fight pain and ease suffering. We stand by the few who suffer from rare diseases and the millions with long-term chronic conditions.

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

Sanofi, Empowering Life

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### 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 and their underlying assumptions, statements regarding plans, objectives, intentions and expectations with respect to future financial results, events, operations, services, product development and potential, and statements regarding future performance. 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, the uncertainties inherent in research and development, future clinical data and analysis, including post marketing, decisions by regulatory authorities, such as the FDA or the EMA, regarding whether and when to approve any drug, device or biological application that may be filed for any such product candidates as well as their decisions regarding labelling and other matters that could affect the availability or commercial potential of such product candidates, the fact that product candidates if approved may not be commercially successful, the future approval and commercial success of therapeutic alternatives, Sanofi’s ability to benefit from external growth opportunities, to complete related transactions and/or obtain regulatory clearances, risks associated*

*with intellectual property and any related pending or future litigation and the ultimate outcome of such litigation, trends in exchange rates and prevailing interest rates, volatile economic and market conditions, cost containment initiatives and subsequent changes thereto, and the impact that COVID-19 will 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. Any material effect of COVID-19 on any of the foregoing could also adversely impact us. This situation is changing rapidly and additional impacts may arise of which we are not currently aware and may exacerbate other previously identified risks. 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, 2020. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.*