

Sanofi, a leader in immune-mediated rare blood disorders, to present latest data at EHA 2022

June 10, 2022. The latest clinical research across Sanofi's portfolio of investigational and approved therapies for immune-mediated rare blood disorders will be presented at the [27th Annual European Hematology Association \(EHA\) Congress](#). Sanofi's goal is to fundamentally redefine the management of these rare and often chronic blood disorders and the company has launched innovative treatments for patients with limited options in cold agglutinin disease (CAD) and acquired thrombotic thrombocytopenic purpura (aTTP).

At EHA, five presentations on CAD will be shared, including two-year follow-up data from the Phase 3 pivotal CARDINAL study that demonstrated the safety and efficacy of Enjaymo™ (sutimlimab-jome). Additional presentations include long-term data from an extension trial of rilzabrutinib, an investigational Bruton's tyrosine kinase (BTK) inhibitor for immune thrombocytopenia (ITP), and three-year safety and efficacy findings for use of Cablivi® (caplacizumab-yhdp) in aTTP.

Karin Knobe, MD, PhD

Global Head of Development, Rare Diseases and Rare Blood Disorders at Sanofi

"Sanofi's commitment to finding solutions where there is significant unmet need is unwavering. Following the FDA's recent approval of Enjaymo™ (sutimlimab) as the first-and-only approved treatment indicated to decrease the need for red blood cell transfusion due to hemolysis in adults with CAD, we are excited to share our latest research in the area in addition to updates regarding our work in ITP and aTTP. The data we're presenting at EHA 22 represent a robust portfolio of potential first-in-class treatments that are providing hope for people living with immune-mediated rare blood disorders."

Cold Agglutinin Disease (CAD) Data Presentations

Enjaymo™ is a first-in-class humanized monoclonal antibody that selectively inhibits the C1 complex of the classical complement pathway, preventing its CAD-associated activation and targeted destruction of healthy red blood cells while leaving the alternative and lectin complement pathways intact. Oral presentations will report two-year follow-up results from the pivotal Phase 3 CARDINAL study, an open-label, single-arm study with a 26-week primary treatment period (Part A), and a 2-year extension (Part B).

Follow-up data support the CARDINAL Part A findings, showing that Enjaymo™ maintained mean hemoglobin >11g/dL and achieved sustained normalization of mean bilirubin in patients after two years, demonstrating the efficacy and safety profile for the therapy in CAD (abstract #S285). Additionally, Enjaymo™ was shown to produce rapid and sustained improvements in Functional Assessment of Chronic Illness Therapy-Fatigue (FACIT-Fatigue) scores and have meaningful impact on patient quality of life based on other patient reported outcomes after two years of treatment (abstract #304). No new safety concerns were identified throughout two years of treatment, and sutimlimab was shown to improve and stabilize critical bloodstream components that are typically altered in patients with CAD.

Immune Thrombocytopenia (ITP) Data Presentation

An oral presentation (abstract #S291) on rilzabrutinib, a potential first-in-class investigational oral Bruton's tyrosine kinase (BTK) inhibitor in development for immune-mediated diseases,

including ITP, will report long-term extension (LTE) findings for a global phase 1/2 trial (NCT03395210) for the treatment of ITP, a rare blood disorder in which platelets are targeted for destruction by the immune system, leading to high bleeding risk and poor quality of life.

Acquired Thrombotic Thrombocytopenic Purpura (aTTP) Data Presentation

Long-term safety and efficacy findings from the post-HERCULES trial (NCT02878603), a three-year prospective follow-up study for patients with acquired thrombotic thrombocytopenic purpura (aTTP) who completed the Phase 3 HERCULES trial (NCT02553317), will also be presented in an oral presentation at EHA 2022 (abstract #S294).

aTTP is a rare, life-threatening autoimmune blood disorder causing excessive blood clot formation and impaired oxygen transport to vital organs.

All Rare Blood Disorders Abstracts:

Cold Agglutinin Disease (CAD)

Inhibition of Complement C1s With Sutimlimab in Patients With Cold Agglutinin Disease (CAD): 2-Year Follow-Up From The CARDINAL Study. Oral Presentation.	Oral Presentation Abstract #S285 Friday, June 10 11:30 – 12:45 CEST
Inhibition of Complement C1s With Sutimlimab in Patients with Cold Agglutinin Disease (CAD): Results Following 9-Week Washout Period In Phase 3 CARDINAL Study (NCT03347396). Poster Presentation.	Poster Presentation Abstract # P1538 Friday, June 10 16:30 – 17:45 CEST
Sutimlimab After Prior Rituximab Use in Patients with Cold Agglutinin Disease (CAD): Pooled Post-hoc Analyses from the CARDINAL and CADENZA Trials. Poster Presentation.	Poster Presentation Abstract # P1532 Friday, June 10 16:30 – 17:45 CEST
Seasonality of Healthcare Resource Utilization Among Cold Agglutinin Disease Among Patients in Denmark: A Retrospective Analysis. Poster Presentation.	Poster Presentation Abstract # P1539 Friday, June 10 16:30 – 17:45 CEST
Sutimlimab, A Complement C1s Inhibitor, Provides Sustained Improvements in Patient-Reported Outcomes in Patients With Cold Agglutinin Disease (CAD): 2 Year Follow-Up From The CARDINAL Study. Oral Presentation.	Oral Presentation Abstract #S304 Saturday, June 11 11:30 – 12:45 CEST

Immune Thrombocytopenia (ITP)

Phase I/II Study of Rilzabrutinib, An Oral Bruton Tyrosine Kinase Inhibitor, in Patients with Immune Thrombocytopenia: Long-Term Follow-Up. Oral Presentation.	Oral Presentation Abstract #S291 Saturday, June 11 11:30 – 12:45 CEST
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Acquired Thrombotic Thrombocytopenic Purpura (aTTP)

Long-term Safety and Efficacy of Caplacizumab for Acquired Thrombotic Thrombocytopenic Purpura (aTTP): The Post-HERCULES Study. Oral Presentation.	Oral Presentation
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About Enjaymo™ (sutimlimab)

Enjaymo is a 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, Enjaymo inhibits the activation of the complement cascade in the immune system and inhibits C1-activated hemolysis in CAD to prevent the abnormal destruction of healthy red blood cells. Enjaymo does not inhibit the lectin and alternative pathways.

Enjaymo was approved by the U.S. Food and Drug Administration in February 2022 as the first and only treatment indicated to decrease the need for red blood cell transfusion due to hemolysis in adults with CAD and Sanofi expects an opinion from the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) in Europe by the end of 2022.

About rilzabrutinib

Rilzabrutinib is an oral Bruton's tyrosine kinase (BTK) 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.

About Cablivi® (caplacizumab)

Cablivi is a von Willebrand Factor (vWF) antibody fragment, which inhibits the interaction between ultra-large vWF multimers and platelets and, therefore, stops the formation of the micro-clots that can form during an acute episode of acquired Thrombotic Thrombocytopenia Purpura. Cablivi was approved in the European Union in August 2018 and in the United States in February 2019.

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

Kate Conway | + 1 508 364 4931 | kate.conway@sanofi.com

Investor Relations

Eva Schaefer-Jansen | + 33 7 86 80 56 39 | eva.schaefer-jansen@sanofi.com

Arnaud Delépine | + 33 6 73 69 36 93 | arnaud.delepine@sanofi.com

Corentine Driancourt | + 33 6 40 56 92 21 | corentine.driancourt@sanofi.com

Felix Lauscher | + 1 908 612 7239 | felix.lauscher@sanofi.com

Priya Nanduri | +1 617 764 6418 | priya.nanduri@sanofi.com

Nathalie Pham | + 33 7 85 93 30 17 | nathalie.pham@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 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, 2021. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.