

Sanofi to present new clinical data reinforcing novel therapies across rare blood disorders at ASH 2022

- Quality of life data for efanesoctocog alfa supports its potential as a transformative, best-in-class therapy for hemophilia A
- Data across hemophilia A, hemophilia B, cold agglutinin disease (CAD), and other conditions featured in 16 presentations

Paris, November 30, 2022. Data from across Sanofi's rare blood disorders franchise will be featured in 16 presentations at the 64th American Society of Hematology (ASH) Annual Meeting & Exposition from December 10-13, 2022.

Karin Knobe, MD, PhD

Global Head of Clinical Development for Rare Diseases and Rare Blood Disorders at Sanofi
"The Phase 3 results presented at ASH across hemophilia and cold agglutinin disease underscore our ambition to launch four rare blood disorder therapies in four years. We started this year strong and look forward to carrying this momentum into 2023 and beyond, with potential treatment advancements we hope will help transform the lives of people living with rare blood disorders."

Quality of life data from the pivotal XTEND-1 Phase 3 study will be presented at ASH evaluating the effect of investigational therapy efanesoctocog alfa on pain and physical functioning for people with hemophilia A. These findings build on key results that were first presented at the 30th International Society of Thrombosis and Haemostasis (ISTH) Congress. Data from the XTEND-1 study supported the biologics licence application (BLA) for efanesoctocog alfa, which is currently under FDA priority review with a target action date of February 28, 2023.

New data to be presented from the ATLAS program for fitusiran, an investigational therapy for the prophylactic treatment of people with hemophilia A or B, with or without inhibitors, will showcase for the first time the hemostatic equivalency of antithrombin lowering in hemophilia A. Hemostatic equivalency is used to better understand how non-factor therapies, such as fitusiran, can help correct the clotting defect in hemophilia. Fitusiran is designed to work by targeting antithrombin, a protein that helps maintain hemostatic balance. Additional fitusiran data will exhibit improvements in health-related quality of life with reduced treatment burden. Qualitative data will outline treatment expectations for people with hemophilia to live an active lifestyle.

In an oral presentation, results from the CADENZA phase 3 study, which supported a supplemental BLA currently under FDA priority review for Enjaymo[®] (sutimlimab-jome), will review the effect of Enjaymo on patient-reported outcomes and quality of life. Presentations will also report long-term clinical data from the CADENZA study as well as information on the COVID-19 vaccine response among people living with cold agglutinin disease (CAD) being treated with Enjaymo. Data from the pivotal CADENZA and CARDINAL Phase 3 studies recently supported the European Commission [approval](#) of Enjaymo.

Additional presentations at ASH will highlight other rare blood disorders. Two posters will explore the prevalence and burden of immune thrombocytopenia (ITP), including cognitive impairment. Data evaluating the response to rilzabrutinib, an investigational therapy, when used as an early treatment option for ITP patients will also be shown. Presentations on acquired thrombotic thrombocytopenic purpura (aTTP) include an analysis of the HERCULES Phase 3 study and a presentation of the MAYARI study design that will assess the efficacy and safety profile of Cablivi[®] (caplacizumab-yhdp) without plasma exchange in an investigational setting. Cablivi is approved in several geographies in combination with plasma exchange and immunosuppression for the treatment of aTTP in adults.

Hemophilia A and B

- Abstract #2468: Efficacy of Efanesoctocog Alfa on Physical Functioning: Results from the XTEND-1 Phase 3 Clinical Trial in Previously Treated Patients with Hemophilia A
 - Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022
- Abstract #2474: Efficacy of Efanesoctocog Alfa on Pain in Patients with Hemophilia A: Results from the XTEND-1 Phase 3 Clinical Trial in Previously Treated Patients with Hemophilia A
 - Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022
- Abstract #3788: A Population Pharmacokinetic (PopPK) Model to Characterize Efanesoctocog Alfa (BIVV001) Factor VIII Activity Levels in Patients with Severe Hemophilia A
 - Poster Presentation: 6-8 pm CT, Monday, December 12, 2022
- Abstract #2472: Development of a Quantitative Systems Pharmacology Model to Explore Hemostatic Equivalency of Antithrombin Lowering
 - Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022
- Abstract #3559: Fitusiran Prophylaxis Improves Health-Related Quality of Life in People with Hemophilia A or B, with or without inhibitors: Results of ATLAS-PPX study
 - Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022
- Abstract #3565: Fitusiran Reaches People's with Hemophilia and Their Caregivers' Treatment Expectations: Qualitative Semi-Structured Interviews of Participants of ATLAS-OLE Trial (Interim analysis)
 - Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022
- Abstract #3566: Patients with Hemophilia and Their Caregivers Prefer Treatment Attributes That Enable a More Active Lifestyle with Lower Risk of Inhibitor Development
 - Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022

Cold Agglutinin Disease

- Abstract #31: Sutimlimab Provides Sustained Improvements in Patient-Reported Outcomes and Quality of Life in Patients with Cold Agglutinin Disease: Open-Label Extension of the Randomized, Phase 3 CADENZA Study
 - Oral Presentation: 9:30-11 am CT, Saturday, December 10, 2022
- Abstract #1201: Sustained Complement C1s Inhibition with Sutimlimab in Patients With Cold Agglutinin Disease Results in Continued Efficacy During Part B of the Randomized Placebo-Controlled Phase 3 CADENZA Study (NCT03347422)
 - Poster Presentation: 5:30-7:30 pm CT, Saturday, December 10, 2022
- Abstract #1208: Concomitant Use Of Sutimlimab and COVID-19 Vaccines In Patients With Cold Agglutinin Disease from the Phase 3 CARDINAL and CADENZA Studies
 - Poster Presentation: 5:30-7:30 pm CT, Saturday, December 10, 2022
- Abstract #2325: Hemolytic Markers, Mortality, and Thromboembolic Events in Cold Agglutinin Disease (CAD): A Retrospective Analysis of the OPTUM Electronic Health Record Database in the United States
 - Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022

Immune Thrombocytopenia

- Abstract #2449: Multirefractory Primary Immune Thrombocytopenia in Adults: Prevalence and Burden. Results from the Carmen-France Registry
 - Poster Presentation: 6-8pm CT, Sunday, December 11, 2022
- Abstract #2450: Clinical Predictors of Response to Rilzabrutinib Therapy in Patients With Immune Thrombocytopenia: Exploratory Analysis of a Phase 1/2 Study

- Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022
- Abstract #3773: Cognitive Impairment Among Patients With Chronic Immune Thrombocytopenia
 - Poster Presentation: 6-8 pm CT, Monday, December 12, 2022

Acquired Thrombotic Thrombocytopenic Purpura

- Abstract #1174: A Phase 3 Study to Evaluate the Efficacy and Safety of Caplacizumab Without First-Line Therapeutic Plasma Exchange in Adults With Immune-Mediated Thrombotic Thrombocytopenic Purpura
 - Poster Presentation: 5:30-7:30 pm CT, Saturday, December 10, 2022
- Abstract #2493: The Role of ADAMTS13 Activity Levels on Disease Exacerbation or Relapse in Patients With Immune-Mediated Thrombotic Thrombocytopenic Purpura: Post Hoc Analysis of the Phase 3 HERCULES and Post-HERCULES Studies
 - Poster Presentation: 6-8 pm CT, Sunday, December 11, 2022

About efanesoctocog alfa

Efanesoctocog alfa is a novel and investigational recombinant factor VIII therapy that is designed to extend protection from bleeds with once-weekly prophylactic dosing for people with hemophilia A. It builds on the innovative Fc fusion technology by adding a region of von Willebrand factor and XTEN[®] polypeptides to extend its time in circulation. It is the first investigational factor VIII therapy that has been shown to break through the von Willebrand factor ceiling, which imposes a half-life limitation on current factor VIII therapies. ALTUVIIIO[®] is the intended trade name of efanesoctocog alfa in the US. Efanesoctocog alfa is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority. Efanesoctocog alfa is currently under FDA review with a target action date of February 28, 2023. The FDA also granted efanesoctocog alfa [Breakthrough Therapy designation](#) in May 2022, – the first factor VIII therapy to receive this recognition – [Fast Track designation](#) in February 2021, and Orphan Drug designation in August 2017. Sanofi and Sobi[®] collaborate on the development of efanesoctocog alfa.

About fitusiran

Fitusiran is an investigational, subcutaneously administered small interference RNA (siRNA) therapeutic in development for the prophylactic treatment of people with hemophilia A or B, with or without inhibitors. Fitusiran is designed to lower antithrombin, a protein that inhibits blood clotting, with the goal of promoting thrombin generation to rebalance hemostasis and prevent bleeds. Fitusiran utilizes Alnylam Pharmaceutical Inc.'s ESC-GalNAc conjugate technology, which enables subcutaneous dosing with increased potency and durability. Fitusiran is currently under clinical investigation and has not been evaluated by any regulatory authority.

About Enjaymo[®] (sutimlimab-jome)

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 cold agglutinin disease (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 (FDA) 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. Sanofi has filed for an sBLA to add CADENZA (BIVV009-04) clinical data into the label and was granted priority review by the FDA in September 2022.

About Cablivi[®] (caplacizumab-yhdp)

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. Use of Cablivi without plasma exchange is currently investigational and has not been evaluated by any regulatory authority.

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 immune thrombocytopenia (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 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

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Sanofi Forward-Looking Statements

This document 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. 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.