

FDA grants priority review of sutimlimab, potential first approved treatment of hemolysis in adult patients with Cold Agglutinin Disease

- * Sutimlimab targets C1-activated hemolysis in cold agglutinin disease (CAD)

PARIS – May 14, 2020 - The U.S. Food and Drug Administration (FDA) has granted priority review of Sanofi’s Biologics License Application (BLA) for sutimlimab for the treatment of hemolysis in adult patients with cold agglutinin disease (CAD). Sutimlimab, an investigational monoclonal antibody, targets the underlying cause of hemolysis in CAD by selectively inhibiting complement C1s.

If approved, sutimlimab would be the first and only approved treatment for these patients. The target action date for the FDA decision is November 13, 2020.

CAD is a chronic autoimmune hemolytic anemia that causes the body’s immune system to mistakenly attack healthy red blood cells and cause their rupture (hemolysis). 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.^{1,2,3} An estimated 5,000 people in the U.S. live with CAD.

“People living with cold agglutinin disease currently have no approved treatment option and experience chronic anemia and profound fatigue, which have a persistent and serious impact on their lives,” said John Reed, M.D., Ph.D., Global Head of Research and Development at Sanofi. *“Results from our 26-week pivotal Phase 3 study clearly demonstrated that sutimlimab had a clinically meaningful effect on complement-mediated hemolysis, which is the cause of anemia and fatigue. If approved, sutimlimab will be the first and only FDA-approved treatment to uniquely address C1-activated hemolysis and help alleviate the chronic disease burden for people with CAD.”*

The BLA submission is based on results from part A (n=24) of the open label, single arm pivotal Phase 3 CARDINAL study in patients with primary CAD. The [data were presented in the Late-Breaking Abstracts Session](#) at the 61st Annual Meeting of the American Society of Hematology and demonstrated sutimlimab met its primary composite efficacy endpoint defined as the proportion of patients who demonstrated an increase from baseline in Hgb level ≥ 2 g/dL or normalization of Hgb level ≥ 12 g/dL at the treatment assessment time point (mean value from weeks 23, 25, and 26) and no blood transfusion from week 5 through week 26. The trial showed sutimlimab also met its secondary endpoints by indicating improvements in disease process, including improvements in hemoglobin,

normalization of bilirubin, and improvements in Functional Assessment of Chronic Illness Therapy-Fatigue Score.

Targeting C1s in the classical complement pathway

Sutimlimab is designed to selectively target and inhibit C1s in the classical complement pathway, which is part of the innate immune system. By blocking C1s, it is thought that sutimlimab halts C1-activated hemolysis in CAD. The inhibition of the classical pathway at C1s aims to retain immune surveillance functional activities of the alternative or lectin complement pathways.

Sanofi is evaluating sutimlimab in the on-going Phase 3 CADENZA trial for CAD patients who have not recently had a blood transfusion and separately, investigating sutimlimab for patients with immune thrombocytopenic purpura. Sutimlimab has been granted Breakthrough Therapy and Orphan Drug designation by the FDA. Sutimlimab is currently under clinical development and its safety and efficacy have not been evaluated by any regulatory authority.

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

Media Relations

Nicolas Kressmann
Tel: +1 (732) 532-5318
Nicolas.Kressmann@sanofi.com

Investor Relations

Felix Lauscher
Tel.: +33 (0)1 53 77 45 45
ir@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, 2019. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.

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 Puneekar, 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.