Sanofi highlights scientific innovations in the field of rare blood disorders at ISTH 2021

* New research from first-in-class marketed and investigational therapies in hemophilia, immune thrombocytopenia and acquired thrombotic thrombocytopenic purpura will be presented

July 2, 2021

Clinical data and new research from Sanofi’s rare blood disorders portfolio of marketed and investigational therapies for people with hemophilia, immune thrombocytopenia (ITP) and acquired thrombotic thrombocytopenic purpura (aTTP) will be presented at the International Society on Thrombosis and Haemostasis (ISTH) 2021 Virtual Congress, July 17-21, 2021.

“Breaking barriers for people living with rare blood disorders requires that we push ourselves to transform the therapeutic landscape,” said Karin Knobe, MD, PhD, Head of Development, Rare Diseases and Rare Blood Disorders at Sanofi. “We deliver on that promise with our robust portfolio and pipeline of novel technologies that are designed to help address the underlying conditions or unmet needs for people with debilitating rare blood disorders. At ISTH, we are sharing data across all our innovative approaches and collectively, the data reinforce how our pipeline and portfolio may unlock new possibilities in the treatment of rare blood disorders.”

Breaking barriers in hemophilia: helping to evolve the treatment paradigm

Hemophilia is a rare, genetic bleeding disorder in which a person’s blood does not clot properly. Despite advances in treatment options in recent years, limitations still exist. Sanofi’s two marketed extended half-life factor replacement therapies shifted a two-decades-old treatment paradigm when launched in 2014. Today, with the molecules in its hemophilia pipeline, Sanofi is focused on delivering potential first-in-class and best-in-class therapies with the goal of bringing efficacy and convenience to a new level for patients.

Fitusiran is an investigational, subcutaneously administered small interference RNA therapy in development for the treatment of people with hemophilia A or B, with or without inhibitors and has the potential to transform treatment as it would be the only prophylactic therapy with as few as six injections per year. Population pharmacokinetic and pharmacodynamic modeling data to characterize the antithrombin (AT) lowering dynamics in hemophilia patients treated with fitusiran will be shared in a poster presentation. These
data support the revised fitusiran dose and dosing regimen implemented in the ongoing adult and adolescent studies.

- **Abstract # PB0526** Fitusiran population pharmacokinetic and pharmacodynamic (PopPK/PD) modeling to support revised dose, dosing regimens and dose mitigation scheme. ePoster

New research will also be presented on Eloctate® [Antihemophilic Factor (Recombinant), Fc Fusion Protein], Sanofi’s extended half-life factor VIII therapy for people with hemophilia A. Sanofi collaborates on the development and commercialization of Eloctate with Sobi, a global biopharmaceutical company.

- **Abstract # PB0518** Treatment patterns and clinical outcomes among patients with hemophilia A treated with factor VIII replacement therapies and nonfactor therapy: an assessment of US real-world data. ePoster

- **Abstract #PB0578** Evaluating the effectiveness of recombinant factor VIII Fc fusion protein (rFVIIIFc) in adolescents and children with hemophilia A in the real world in Japan: interim analysis of the multicenter, observational Fc Adolescent and Children Treatment study (FACTs). ePoster

- **Abstract # PB0522** Final results of RelITirate - a prospective study of rescue immune tolerance induction (ITI) with recombinant factor VIII Fc (rFVIIIFc) in patients who have failed previous ITI attempts (Joint with Sobi). ePoster

- **Abstract # OC48.2** Transplacental delivery of recombinant Fc-fused factor VIII (rFVIIIFc) in FVIII-deficient mice (ESC). Investigator Sponsored Study. Oral presentation

**Charting a new course in ITP and aTTP**

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 patients failing to maintain their platelet counts with the current therapies.

Rilzabrutinib is an investigational Bruton’s tyrosine kinase (BTK) inhibitor incorporating Sanofi’s TAILORED COVALENCY® technology that is in development for the treatment of ITP. Interim Phase 1/2 study data will be presented at ISTH.

- **Abstract # OC72.2** Phase I/II ongoing study of rilzabrutinib, an oral Bruton tyrosine kinase (BTK) inhibitor, in immune thrombocytopenia (ITP): extended follow-up and long-term analyses with optimal dose. Oral Presentation

Acquired TTP is a rare, life-threatening, autoimmune blood clotting disorder and is considered a medical emergency. New research will be shared on Cablivi®
(caplacizumab-yhdp), a von Willebrand Factor-directed antibody fragment treatment indicated for use in combination with plasma exchange and immunosuppressive therapy for adult patients with aTTP.

- **Abstract # PB0838** Caplacizumab rapidly inhibits VWF-platelet interaction: pharmacodynamic data from healthy volunteers and patients with aTTP. ePoster

- **Abstract # PB0851** PK/PD modeling and simulations highlight the importance of the intravenous loading dose and daily dosing regimen with caplacizumab for patients with aTTP. ePoster

**About the Sanofi and Sobi collaboration:** Sanofi and Sobi collaborate on the development and commercialization of Eloctate®/Elocta® and Alprolix®. Sobi has final development and commercialization rights in the Sobi territory (essentially Europe, North Africa, Russia, and most Middle Eastern markets). Sanofi has final development and commercialization rights in North America and all other regions in the world excluding the Sobi territory and has manufacturing responsibility for Eloctate/Elocta and Alprolix. In September 2019, Sobi exercised early opt-in for the development and commercialization of efanesoctocog alfa, an investigational factor VIII therapy with the potential to provide extended protection from bleeds with once-weekly dosing for people with hemophilia A.

**Editor’s Note:**

**About fitusiran:** Fitusiran is an investigational, subcutaneously administered, small interference RNA therapeutic in development for the prophylaxis treatment of people with hemophilia A or B, with or without inhibitors. Fitusiran is designed to target antithrombin, a protein that inhibits blood clotting, with the goal of promoting sufficient 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 Eloctate:** Eloctate® [Antihemophilic Factor (Recombinant), Fc Fusion Protein] is a recombinant clotting factor therapy developed for hemophilia A using Fc fusion technology to prolong circulation in the body. It is engineered by fusing factor VIII to the Fc portion of immunoglobulin G subclass 1, or IgG1 (a protein commonly found in the body), enabling Eloctate to use a naturally occurring pathway to extend the time the therapy remains in the body.

Eloctate is marketed by Sanofi in the United States, Japan and Canada. It is also approved in Australia, New Zealand, Brazil and other countries, and Sanofi has marketing rights in these regions. It is also approved as Elocta® in the European Union, Switzerland, Iceland, Liechtenstein, Norway and other countries where it is marketed by Sobi.

**About rilzabrutinib:** Rilzabrutinib is an oral, peripheral Bruton’s tyrosine kinase inhibitor incorporating Sanofi’s TAILORED COVALENCY® technology being investigated for the
treatment of immune-mediated diseases, including immune thrombocytopenia. BTK is involved in innate and adaptive immune responses and is a signaling molecule in immune mediated diseases. Rilzabrutinib has the potential to target the underlying disease pathogenesis of ITP and has not been shown to alter platelet aggregation.

The clinical significance of these mechanisms is currently under investigation.

Rilzabrutinib is currently under clinical investigation and has not been evaluated 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-related disease. Three additional Phase 2 studies in immunological diseases are planned to start in 2021.

**About Cablivi:** 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.

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*TAILORED COVALENCY is a registered trademark of Principia Biopharma Inc., a Sanofi Company*

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**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 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.