**Press Release**

# *ALTUVIIIO™ late-breaking data at ISTH demonstrates highly effective bleed protection in children with severe hemophilia A with once-weekly dosing*

- XTEND-Kids data confirm the efficacy and safety profile of ALTUVIIIO with simple, weekly 50 IU/kg dosing for both adults and children
- ALTUVIIIO's expanding evidence of a first and best-in-class profile supports Sanofi's commitment to delivering paradigm shifting therapies for Rare Diseases

**Paris, June 25, 2023**. Pivotal data from the Phase 3 XTEND-Kids study evaluating ALTUVIIIO™ [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein] once-weekly prophylaxis, a first-in-class, high-sustained factor VIII replacement therapy, in previously treated patients younger than 12 years of age with severe hemophilia A were presented today in a late-breaking session at the Annual Meeting of the International Society on Thrombosis and Haemostasis (ISTH) in Montreal, Canada.

The oral presentation detailed results from the XTEND-Kids study and confirmed that ALTUVIIIO met the primary endpoint with no inhibitor development to factor VIII detected, and key secondary endpoints including annualized bleeding rate (ABR) and maintenance of factor VIII activity above pre-specified levels.

In the pediatric population, clearance of administered factor concentrates in the blood is greater than in adults, often meaning injections are needed 2-4 times per week using standard (SHL) or extended half-life (EHL) factor VIII products. These data confirm that a once-weekly 50 IU/kg dose of ALTUVIIIO provides highly effective bleed protection in both children and adults and can be used across clinical scenarios.

Hemophilia A is a rare, lifelong condition in which the ability of a person's blood to clot properly is impaired, leading to excessive bleeds and spontaneous bleeds into joints that can result in joint damage and chronic pain, and potentially impact quality of life. The severity of hemophilia is determined by the level of clotting factor activity in a person's blood, and there is a negative correlation between risk of bleeding and factor activity levels.

ALTUVIIIO is a first-in-class, high-sustained factor VIII replacement therapy approved in February 2023 by the US Food and Drug Administration (FDA) for routine prophylaxis and on-demand treatment to control of bleeding episodes, as well as perioperative management (surgery) for adults and children with hemophilia A. Granted Breakthrough Therapy designation by the FDA in May 2022 — the first factor VIII therapy to receive this designation — ALTUVIIIO also received Fast Track designation in February 2021 and Orphan Drug designation in 2017. The European Commission granted Orphan Drug designation in June 2019, and the European Medicines Agency accepted the Marketing Authorization Application (MAA) for efanesoctocog alfa in May 2023.

***Lynn Malec, MD***
Medical Director of Comprehensive Center for Bleeding Disorders and Associate Investigator at The Versiti Blood Research Institute, and Associate Professor of Medicine and Pediatrics at The Medical College of Wisconsin
*"The results from XTEND-Kids mark an important breakthrough as we strive for optimized bleed protection as the standard of care. Achieving high-sustained factor activity with once weekly dosing means a freedom from the tradeoffs between treatment burden and efficacy we often see in treating severe hemophilia A. The reliable and consistent bleed protection ALTUVIIIO provides offers*

*confidence for children living with hemophilia and their families to manage hemophilia with less worry."*

***Karin Knobe, MD, PhD***
Therapeutic Area Head, Rare Diseases and Rare Blood Disorders, Sanofi
*"In an effort to reduce their risk of bleeding episodes, many children living with hemophilia A are currently limited in their ability to fully participate in daily activities. This burden is compounded by the challenge of administering prophylactic treatments intravenously multiple times a week. Today's XTEND-Kids results reinforce the ability of ALTUVIIIO to provide effective bleed protection with once weekly dosing and reinforce our commitment to developing new treatment options designed to redefine the standard of care for people living with rare blood disorders."*

### *Key Results*

The Phase 3 XTEND-Kids study (NCT04759131) was an open-label, non-randomized interventional study of the safety, efficacy, and pharmacokinetics of once-weekly ALTUVIIIO in previously treated patients younger than 12 years of age with severe hemophilia A. Patients (n=74) received once-weekly ALTUVIIIO prophylaxis (50 IU/kg) for 52 weeks.

- Development of Factor VIII inhibitors was not detected (0% [95% confidence interval (CI)] 0–4.9]).
- Median (interquartile range) and mean ABRs (95% CI) were 0.00 (0.00–1.02) and 0.89 (0.56–1.42), respectively
- 64% of patients had zero bleeding episodes, 82% of patients had zero joint bleeds and 88% of patients had zero spontaneous bleeds
- In this study, ALTUVIIIO was well-tolerated and demonstrated a safety profile similar to the XTEND-1 trial, confirming safety and efficacy in both adults and children.
- No serious allergic reactions, anaphylaxis, or embolic or thrombotic events were reported. The most common treatment-emergent adverse events (>10%) were SARS-CoV-2 test positive, upper respiratory tract infection, and fever (pyrexia). No adverse events led to treatment discontinuation.

### *About ALTUVIIIO™*

ALTUVIIIO [Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein-ehtl] is a first-in-class high-sustained factor VIII therapy that is designed to extend protection from bleeds with once-weekly prophylactic dosing for adults and children with hemophilia A. In adults and adolescents, ALTUVIIIO has a 3 to 4 fold longer half-life relative to standard and extended half-life factor VIII products, providing high-sustained factor activity levels within normal to near-normal range, allowing for once weekly administration in both children and adults. ALTUVIIIO is the first factor VIII therapy that has been shown to break through the von Willebrand factor ceiling, which imposes a half-life limitation on earlier generation factor VIII therapies. ALTUVIIIO builds on the innovative Fc fusion technology by adding a region of von Willebrand factor and XTEN® polypeptides to extend its time in circulation.

### *About the XTEND Clinical Programs*

The XTEND clinical program is comprised of two Phase 3 trials in hemophilia A: XTEND-1 in people 12 years or older and XTEND-Kids in children younger than 12 years old. There is also an ongoing extension study (XTEND-ed).

The Phase 3 XTEND-1 study (NCT04161495) was an open-label, non-randomized interventional study assessing the safety, efficacy, and pharmacokinetics of once-weekly ALTUVIIIO in people 12 years of age or older (n=159) with severe hemophilia A who were previously treated with factor VIII replacement therapy. The study consisted of two parallel treatment arms — the prophylaxis Arm A (n=133), in which patients who had received prior factor VIII prophylaxis were treated with once-weekly intravenous ALTUVIIIO prophylaxis (50 IU/kg) for 52 weeks, and the on-demand Arm B (n=26), in which patients who had received prior on-demand factor VIII therapy began with 26 weeks of on-demand ALTUVIIIO (50 IU/kg), then switched to once-weekly prophylaxis with ALTUVIIIO (50 IU/kg) for an additional 26 weeks.

The primary efficacy endpoint of XTEND-1 was the mean annualized bleeding rate (ABR) in Arm A, and the key secondary endpoint was an intra-patient comparison of ABR during the ALTUVIIIO weekly prophylaxis treatment period versus the prior factor VIII prophylaxis ABR for a subset of participants in Arm A who had participated in a previous observational study (Study 242HA201/OBS16221).

The XTEND-Kids study (NCT04759131) was an open-label, non-randomized interventional study of the safety, efficacy, and pharmacokinetics of once-weekly ALTUVIIIO in previously treated patients younger than 12 years of age with severe hemophilia A. Patients received once-weekly ALTUVIIIO prophylaxis (50 IU/kg) for 52 weeks. The primary endpoint was the occurrence of inhibitor development.

### *About the Sanofi and Sobi collaboration*

Sobi and Sanofi collaborate on the development and commercialization of Alprolix® and Elocta®/Eloctate®. The companies also collaborate on the development and commercialization of efanesoctocog alfa, or ALTUVIIIO in the US. 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.

### *About Sobi®*

Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare and debilitating diseases. Providing reliable access to innovative medicines in the areas of haematology, immunology and specialty care, Sobi has approximately 1,600 employees across Europe, North America, the Middle East, Asia and Australia. In 2022, revenue amounted to SEK 18.8 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. More about Sobi at sobi.com, LinkedIn and YouTube.

### *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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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 pandemics or other global crises may 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, 2022. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.