

FDA grants priority review to efanesoctocog alfa for people with hemophilia A

- The FDA decision date for efanesoctocog alfa, an investigational factor VIII therapy, is set for February 28, 2023
- Priority review based on pivotal data from the XTEND-1 Phase 3 study
- Efanesoctocog alfa delivers high sustained factor activity levels in the normal to near-normal range for the majority of the week with once weekly prophylaxis dosing, providing higher protection for longer

Paris and Stockholm – August 30, 2022 – The U.S. Food and Drug Administration (FDA) has accepted for priority review the Biologics License Application (BLA) for efanesoctocog alfa (BIVV001) for the treatment of hemophilia A, a rare and life-threatening bleeding disorder. The target action date for the FDA decision is February 28, 2023. Sanofi and Sobi® collaborate on the development and commercialization of efanesoctocog alfa.

Steve Pipe, MD

Professor and Director of Pediatric Hemophilia and Coagulation Disorders Program, University of Michigan

“Factor therapy remains a cornerstone of hemophilia treatment, but innovation has been needed in this area to address challenges related to bleed protection and cumbersome treatment regimens. If approved, efanesoctocog alfa can deliver close to normal factor activity levels for the majority of the week, potentially offering a new tier of protection. Such therapeutic benefits would represent important advances in unmet medical needs for people with hemophilia A and may transform the prophylactic treatment landscape.”

The BLA is supported by data from the pivotal XTEND-1 Phase 3 study. Results were recently [presented](#) at the 30th International Society of Thrombosis and Haemostasis Congress. The data demonstrate a clinically meaningful prevention of bleeds and superiority to prior factor prophylaxis based on an intra-patient comparison. Efanesoctocog alfa was well-tolerated, and inhibitor development to factor VIII was not detected. The most common treatment-emergent adverse events (>5% of participants overall) were headache, arthralgia, fall, and back pain.

Dietmar Berger, MD, PhD

Global Head of Development and Chief Medical Officer at Sanofi

“The results from the pivotal XTEND-1 Phase 3 study demonstrate efanesoctocog alfa’s ability to reduce annualized bleeding rates, which supports its potential as a therapy with best-in-disease efficacy. We look forward to working closely with the FDA during the review process as we aim to bring this novel therapy to the hemophilia A community.”

The FDA grants priority review to therapies that have the potential to provide significant improvements in the treatment, diagnosis, or prevention of serious conditions. Efanesoctocog alfa received [Breakthrough Therapy designation](#) from the FDA in May 2022 and it is the first factor VIII therapy to receive this recognition. The FDA also granted efanesoctocog alfa Orphan Drug designation in August 2017 and [Fast Track designation](#) in February 2021.

Regulatory submission in the EU will follow availability of data from the ongoing XTEND-Kids pediatric study, with both events expected in 2023. The European Commission granted efanesoctocog alfa Orphan Drug designation in June 2019.

About Phase 3 XTEND-1 Study (NCT04161495)

The Phase 3 XTEND-1 study (NCT04161495) was an open-label, non-randomized interventional study assessing the safety, efficacy and pharmacokinetics of once-weekly efanesoctocog alfa 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 efanesoctocog alfa 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 26 weeks of on-demand efanesoctocog alfa (50 IU/kg), then switched to once-weekly prophylaxis (50 IU/kg) for an additional 26 weeks.

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

About hemophilia A

Hemophilia A is a rare, genetic disorder in which the ability of a person's blood to clot is impaired due to a lack of factor VIII. Hemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. People with hemophilia can experience bleeding episodes that can cause pain, irreversible joint damage and life-threatening hemorrhages. Factor replacement therapy remains a cornerstone of care and can be used across multiple treatment scenarios.

About efanesoctocog alfa (BIVV001)

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. Efanesoctocog alfa is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority.

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. 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 specialized international biopharmaceutical company transforming the lives of people with rare diseases. Providing sustainable 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 and Asia. In 2021, revenue amounted to SEK 15.5 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.

Sanofi Contacts:

Media Relations

Sandrine Guendoul | + 33 6 25 09 14 25 | sandrine.guendoul@sanofi.com

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

Sobi Contacts:

Media Relations

For Sobi Media contacts, click [here](#).

Investor Relations

For details on how to contact the Sobi Investor Relations Team, click [here](#).

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.