

# *FDA grants efanesoctocog alfa Breakthrough Therapy designation for hemophilia A*

- Efanesoctocog alfa is the first factor VIII therapy to be awarded Breakthrough Therapy designation by the FDA
- Designation is based on XTEND-1 Phase 3 study data demonstrating a clinically meaningful prevention of bleeds and superiority in prevention of bleeding episodes compared to prior prophylaxis factor treatment
- Efanesoctocog alfa is a novel and investigational factor VIII therapy designed to provide normal to near-normal factor activity levels for the majority of the week in a once-weekly prophylactic treatment regimen

**Paris and Stockholm – June 1, 2022** – The United States Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to efanesoctocog alfa (BIVV001) for the treatment of people with hemophilia A, a rare and life-threatening bleeding disorder, based on data from the pivotal [XTEND-1 Phase 3 study](#). Sanofi and Sobi® collaborate on the development and commercialization of efanesoctocog alfa.

Breakthrough Therapy designation is designed to expedite the development and review of drugs in the US that target serious or life-threatening conditions. Drugs qualifying for this designation must show preliminary clinical evidence that the drug may demonstrate a substantial improvement on clinically significant endpoints over available therapies.

### ***John Reed, MD, PhD***

Global Head of Research and Development at Sanofi

*“The Breakthrough Therapy designation highlights efanesoctocog alfa’s potential to transform treatment for people with hemophilia A by providing higher protection for longer duration. This potential new class of factor VIII therapy represents how we are boldly advancing science to address unmet needs for the hemophilia community. We are excited to work with regulatory authorities during the filing and review of this innovative therapy.”*

### ***Anders Ullman, MD, PhD***

Head of Research and Development and Chief Medical Officer at Sobi

*“This designation supports the innovation of efanesoctocog alfa and acknowledges its potential to fulfill an unmet medical need for people living with hemophilia A. We are committed to transforming lives for people living with rare diseases, and this is a testament to the medical innovation that science can bring.”*

Topline results from the pivotal XTEND-1 Phase 3 study demonstrate efanesoctocog alfa met the primary endpoint, showing a clinically meaningful prevention of bleeds in people with severe hemophilia A over a 52-week period. Importantly, the key secondary endpoint was also met, demonstrating that efanesoctocog alfa was superior to prior prophylactic factor VIII replacement therapy in preventing bleeding events 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.

Data from the XTEND-1 Phase 3 study are expected to be shared at an upcoming medical meeting, and those data will serve as the basis for submission to FDA mid-year 2022. The FDA granted efanesoctocog alfa Orphan Drug designation in August 2017 and [Fast Track designation](#) in February 2021. The European Commission also granted efanesoctocog alfa Orphan Drug designation in June 2019. Regulatory submission in the EU will follow availability of data from the ongoing XTEND-Kids pediatric study, expected in 2023.

Hemophilia A occurs in about one in 5,000 male births annually, and more rarely in females. It is a lifelong condition in which the ability of a person's blood to clot is impaired due to a coagulation factor deficiency. People with hemophilia can experience bleeding episodes that can cause pain, irreversible joint damage, and life-threatening hemorrhages. Unmet medical needs remain for people with hemophilia to strengthen protection, reduce treatment burden, and improve quality of life.

### *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, an investigational factor VIII therapy with the potential to provide high sustained factor activity levels with once-weekly dosing for people with hemophilia A. 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](https://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](mailto:sandrine.guendoul@sanofi.com)

**Sally Bain** | + 1 617 834 6026 | [sally.bain@sanofi.com](mailto:sally.bain@sanofi.com)

##### *Investor Relations*

**Eva Schaefer-Jansen** | + 33 7 86 80 56 39 | [eva.schaefer-jansen@sanofi.com](mailto:eva.schaefer-jansen@sanofi.com)

**Arnaud Delépine** | + 33 6 73 69 36 93 | [arnaud.delepine@sanofi.com](mailto:arnaud.delepine@sanofi.com)

**Corentine Driancourt** | + 33 6 40 56 92 21 | [corentine.driancourt@sanofi.com](mailto:corentine.driancourt@sanofi.com)

**Felix Lauscher** | + 1 908 612 7239 | [felix.lauscher@sanofi.com](mailto:felix.lauscher@sanofi.com)

**Priya Nanduri** | +1 617 764 6418 | [priya.nanduri@sanofi.com](mailto:priya.nanduri@sanofi.com)

**Nathalie Pham** | + 33 7 85 93 30 17 | [nathalie.pham@sanofi.com](mailto: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.