

## Sanofi announces positive long-term efficacy and safety data for fitusiran from interim analysis of Phase 2 extension study in people with hemophilia A and B, with or without inhibitors

- \* Fitusiran, a novel RNAi therapy in development, has the potential to transform the treatment of hemophilia with a monthly, subcutaneous treatment for people with hemophilia A and B, with or without inhibitors
- \* Long-term exploratory data show prophylactic treatment with fitusiran provides a sustained reduction in annual bleed rates in moderate to severe hemophilia A and B patients, with or without inhibitors.

**Paris – June 19, 2020** – New data exploring the efficacy and safety of fitusiran, an investigational once-monthly, subcutaneously administered RNA interference (RNAi) therapy for the treatment of hemophilia A and B, with or without inhibitors, were shared today in a late-breaking presentation at the World Federation of Hemophilia Virtual Summit.

Long-term interim results from the Phase 2 open-label extension (OLE) study reinforce fitusiran's potential to restore hemostatic balance and to lower annualized bleed rates (ABRs) over a period up to 57 months.

*“These new interim data support the potential of fitusiran to have a transformative impact on hemophilia management with the aim to provide patients with consistent bleed protection and only once monthly subcutaneous dosing,” said Dietmar Berger, Global Head of Development, Sanofi. “We are continuing to advance our portfolio of factor and non-factor therapies that could offer people with hemophilia a broad range of therapeutic options to fit their individual needs. We continue to investigate the clinical profile of fitusiran in our Phase 3 ATLAS program with results expected in the first half of 2021 and look forward to offering this novel therapy to patients globally.”*

### The Phase 2 open-label extension study

The Phase 2 OLE study evaluates the long-term efficacy and safety of fitusiran in patients with moderate or severe hemophilia A and B, with or without inhibitors, who had participated in a previous study of fitusiran. This data evaluated 34 enrolled patients who received monthly fixed 50 mg or 80 mg doses of fitusiran and were followed for a period

up to 4.7 years, with a median exposure of 2.6 years. At the data cutoff (March 10, 2020), interim results showed:

- Monthly subcutaneous dosing with fitusiran, in patients with hemophilia A and B, with or without inhibitors, demonstrated sustained antithrombin lowering (a reduction of around 75% from baseline), resulting in median peak thrombin values at the lower end of the range observed in healthy volunteer participants.
- Low overall median ABR of 0.84. The median ABR in the non-inhibitor subgroup was 1.01 compared to pre-study median ABRs of 2.0 for patients previously on prophylactic treatment and 12.0 for patients previously on demand. In the inhibitor subgroup, median ABRs were 0.44 compared to pre-study median ABRs of 42.0.
- Low overall spontaneous bleeds (overall median of 0.38). Median spontaneous ABRs by subgroup were 0.33 (non-inhibitor) and 0.39 (inhibitors).
- No anti-drug antibody formation was detected

As of the data cut on March 10, 2020, fitusiran was generally well tolerated. Reported serious adverse events with fitusiran included an event of atrial thrombosis and an event of increased liver transaminases. One death occurred in the study in 2017; this was due to cerebral venous sinus thrombosis initially diagnosed as subarachnoid hemorrhage after which the bleed management guidelines were updated in December 2017. The most commonly reported adverse events ( $\geq 5$  patients) included an increase in alanine aminotransferase (29%), headache (27%), injection site erythema (21%), nasopharyngitis (21%), upper respiratory tract infection (18%), diarrhea (18%), arthralgia (18%), back pain (18%), and an increase in transaminases (15%).

### **A novel investigational approach for hemophilia A and B**

Hemophilia A and B are characterized by hemostatic imbalance due to factor VIII and IX deficiency, respectively, resulting in insufficient thrombin generation. All therapies for hemophilia aim to restore hemostatic balance and improve thrombin generation.

Fitusiran is an investigational, once-monthly, subcutaneously administered RNA interference therapeutic in development for the treatment of people with hemophilia A and 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 naturally rebalance hemostasis and prevent bleeds. Fitusiran utilizes Alnylam'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 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.

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#### **Sanofi Forward-Looking Statements**

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