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Alnylam and Sanofi Genzyme to Present New Clinical Trial Results at the International Society of Thrombosis and Haemostasis (ISTH) Congress

- New Data from an Ongoing Phase 2 Open-label Extension Study with Fitusiran in Patients with Hemophilia A and B, With or Without Inhibitors-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- [Alnylam Pharmaceuticals, Inc.](#) (Nasdaq:ALNY), the leading RNAi therapeutics company and [Sanofi Genzyme](#), the specialty care global business unit of [Sanofi](#), announced that new results from an ongoing Phase 2 open-label extension (OLE) study with the investigational therapeutic, fitusiran, in patients with hemophilia A and B, with or without inhibitors, will be presented in an oral presentation at the International Society of Thrombosis and Haemostasis (ISTH) Congress, being held from July 8 - 13, 2017 in Berlin, Germany. In addition, preclinical data further elucidating fitusiran's novel mechanism of action will also be presented at the meeting.

"The data being presented at ISTH demonstrate the growing body of clinical and nonclinical evidence that support fitusiran as a potential treatment approach for patients with hemophilia A and B, with and without inhibitors," said Akin Akinc, Ph.D., Alnylam's Vice President and General Manager, Fitusiran.

"We are committed to furthering research to address the unmet needs of people living with hemophilia," said Baisong Mei, M.D., Ph.D., Sanofi's Senior Global Project Head, Alnylam Portfolio. "We look forward to having the data presented at ISTH regarding the safety and clinical activity of fitusiran from this ongoing Phase 2 OLE study."

Alnylam and Sanofi Genzyme presentations include:

- | Fitusiran, an Investigational RNAi Therapeutic Targeting Antithrombin for the Treatment of Hemophilia: Interim Results from a Phase 2 Extension Study in Patients with Hemophilia A or B with and without Inhibitors
Presenter: K. John Pasi, Royal London Haemophilia Centre, United Kingdom
Session: Abstract Symposia Sessions, Clinical Research and Outcomes in Hemophilia
Date/Time: Monday, July 10, 8:45 - 10:15 a.m. ET (2:45 - 4:15 p.m. CEST)
- | Development of a Pharmacokinetic-Pharmacodynamic (PK-PD) Model of Fitusiran, an Investigational RNAi Therapeutic Targeting Antithrombin for the Treatment of Hemophilia in Patients with and without Inhibitors
Presenter: Husain Attarwala, Alnylam Pharmaceuticals, United States
Session: Poster Session, Exhibition Hall 4.2
Date/Time: Tuesday, July 11, 6:00 - 7:15 a.m. ET (12:00 - 2:15 p.m. CEST)
- | Measurement of Thrombin Generation in Conditions of Low Antithrombin
Presenter: Peter Geisen, Thrombinoscope BV, Maastricht, The Netherlands
Session: Poster Session, Exhibit Hall 6.2
Date/Time: Tuesday, July 11, 6:00 - 7:15 a.m. ET (12:00 - 2:15 p.m. CEST)
- | The Combined Use of Bypassing Agents with Antithrombin Reduction in Plasma of Hemophilia A and B Patients with Inhibitors
Presenter: Gili Kenet, National Hemophilia Center, Sheba Medical Center, Israel
Session: Poster Session, Exhibit Hall 4.2
Date/Time: Tuesday, July 11, 6:00 - 7:15 a.m. ET (12:00 - 2:15 p.m. CEST)

Alnylam will also be presenting nonclinical data on ALN-F12, a development candidate targeting Factor XII (ALN-F12) for the treatment of hereditary angioedema and the prevention of thrombosis.

- | Reduction of Hepatic Factor XII Expression in Mice by ALN-F12 Inhibits Thrombosis without Increasing Bleeding Risk
Presenter: Jingxuan Liu, Alnylam Pharmaceuticals, United States
Session: Abstract Symposia Sessions, Bleeding Management Under Oral Anticoagulation
Date/Time: Monday, July 10, 8:45 - 10:15 a.m. ET (2:45 - 4:15 p.m. CEST)

About Hemophilia

Hemophilia is a hereditary bleeding disorder characterized by an underlying defect in the ability to generate adequate levels of thrombin needed for effective clotting, thereby resulting in recurrent bleeds into joints, muscles, and major internal organs. There are approximately 200,000 persons diagnosed worldwide with hemophilia A and hemophilia B.

Standard treatment for people with hemophilia currently involves replacement of the deficient clotting factor either as prophylaxis or "on-demand" therapy which can lead to a temporary restoration of thrombin generation capacity. However, as many as one third of people with severe hemophilia A will develop a neutralizing antibody to their replacement factor - a very serious complication; individuals with these 'inhibitors' become refractory to standard replacement factor therapy.

About Fitusiran

Fitusiran is an investigational, once-monthly, subcutaneously administered RNAi therapeutic targeting antithrombin (AT) for the treatment of hemophilia A and B, with and without inhibitors. Fitusiran also has the potential to be used for rare bleeding disorders. Fitusiran is designed to lower levels of AT with the goal of promoting sufficient thrombin generation to restore hemostasis and prevent bleeding. Fitusiran utilizes Alnylam's ESC-GalNAc conjugate technology, which enables subcutaneous dosing with increased potency and durability. The clinical significance of this technology is under investigation.

The safety and efficacy of fitusiran have not been evaluated by the U.S. Food and Drug Administration or any other health authority.

Alnylam - Sanofi Genzyme Alliance

In January 2014, Alnylam and Sanofi Genzyme, the specialty care global business unit of Sanofi, formed an alliance to accelerate the advancement of RNAi therapeutics as a potential new class of innovative medicines for patients around the world with rare genetic diseases. The alliance enables Sanofi Genzyme to expand its rare disease pipeline with Alnylam's novel RNAi technology and provides access to Alnylam's R&D engine, while Alnylam benefits from Sanofi Genzyme's proven global capabilities to advance late-stage development and, upon commercialization, accelerate market access for these promising genetic medicine products.

In November 2016, Sanofi Genzyme elected to co-develop (through Sanofi R&D) and co-commercialize fitusiran in the United States, Canada and Western Europe, in addition to commercializing fitusiran in its rest of world territories.

About RNAi

RNAi (RNA interference) is a revolution in biology, representing a breakthrough in understanding protein synthesis in cells, and a completely new approach to drug discovery and development. Its discovery has been heralded as "a major scientific breakthrough that happens once every decade or so," and represents one of the most promising and rapidly advancing frontiers in biology and drug discovery today which was awarded the 2006 Nobel Prize for Physiology or Medicine. RNAi is a natural process of gene silencing that occurs in organisms ranging from plants to mammals. By harnessing the natural biological process of RNAi occurring in our cells, the creation of a major new class of medicines, known as RNAi therapeutics, is on the horizon. Small interfering RNA (siRNA), the molecules that mediate RNAi and comprise Alnylam's RNAi therapeutic platform, target the cause of diseases by potently silencing specific mRNAs, with the goal of preventing disease-causing proteins from being made.

About Alnylam Pharmaceuticals

Alnylam (Nasdaq: ALNY) is leading the translation of RNA interference (RNAi) into a whole new class of innovative medicines with the potential to transform the lives of patients who have limited or inadequate treatment options. Based on Nobel Prize-winning science, RNAi therapeutics represent a powerful, clinically validated approach for the treatment of a wide range of debilitating diseases. Founded in 2002, Alnylam is delivering on a bold vision to turn scientific possibility into reality, with a robust discovery platform and deep pipeline of investigational medicines, including three product candidates that are in late-stage development or will be in 2017. Looking forward, Alnylam will continue to execute on its "Alnylam 2020" strategy of building a multi-product, commercial-stage biopharmaceutical company with a sustainable pipeline of RNAi-based medicines. For more information about our people, science and pipeline, please visit www.alnylam.com and engage with us on Twitter at @Alnylam.

About Sanofi

Sanofi, a global healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs. Sanofi is organized into five global business units: Diabetes and Cardiovascular, General Medicines and Emerging Markets, Sanofi Genzyme, Sanofi Pasteur and Consumer Healthcare.

Sanofi Genzyme focuses on developing specialty treatments for debilitating diseases that are often difficult to diagnose and treat, providing hope to patients and their families. Learn more at www.sanofigenzyme.com.

Alnylam Forward Looking Statements

Various statements in this release concerning Alnylam's future expectations, plans and prospects, including without

limitation, Alnylam's views with respect to the potential for RNAi therapeutics, including fitusiran and ALN-F12, its plans regarding the reporting of clinical data from its clinical trials of fitusiran and its preclinical studies of ALN-F12, and its expectations regarding its STAR pipeline growth strategy and its "Alnylam 2020" guidance for the advancement and commercialization of RNAi therapeutics, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. Actual results and future plans may differ materially from those indicated by these forward-looking statements as a result of various important risks, uncertainties and other factors, including, without limitation, Alnylam's ability to discover and develop novel drug candidates and delivery approaches, successfully demonstrate the efficacy and safety of its product candidates, the pre-clinical and clinical results for its product candidates, which may not be replicated or continue to occur in other subjects or in additional studies or otherwise support further development of product candidates for a specified indication or at all, actions or advice of regulatory agencies, which may affect the design, initiation, timing, continuation and/or progress of clinical trials or result in the need for additional pre-clinical and/or clinical testing, delays, interruptions or failures in the manufacture and supply of its product candidates, obtaining, maintaining and protecting intellectual property, Alnylam's ability to enforce its intellectual property rights against third parties and defend its patent portfolio against challenges from third parties, obtaining and maintaining regulatory approval, pricing and reimbursement for products, progress in establishing a commercial and ex-United States infrastructure, competition from others using technology similar to Alnylam's and others developing products for similar uses, Alnylam's ability to manage its growth and operating expenses, obtain additional funding to support its business activities, and establish and maintain strategic business alliances and new business initiatives, Alnylam's dependence on third parties for development, manufacture and distribution of products, the outcome of litigation, the risk of government investigations, and unexpected expenditures, as well as those risks more fully discussed in the "Risk Factors" filed with Alnylam's most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) and in other filings that Alnylam makes with the SEC. In addition, any forward-looking statements represent Alnylam's views only as of today and should not be relied upon as representing its views as of any subsequent date. Alnylam explicitly disclaims any obligation, except to the extent required by law, to update any forward-looking statements.

None of Alnylam's investigational therapeutics have been approved by the U.S. Food and Drug Administration, European Medicines Agency, or any other regulatory authority and no conclusions can or should be drawn regarding the safety or effectiveness of these therapeutics.

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 absence of guarantee that the product candidates if approved will be commercially successful, the future approval and commercial success of therapeutic alternatives, Sanofi's ability to benefit from external growth opportunities 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 conditions, the impact of cost containment initiatives and subsequent changes thereto, the average number of shares outstanding as well as those 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, 2016. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.

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