Alnylam and Sanofi Submit Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for Patisiran for the Treatment of Hereditary ATTR (hATTR) Amyloidosis

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– Patisiran MAA to be Reviewed Under Accelerated Assessment –

CAMBRIDGE, Mass. & PARIS--(BUSINESS WIRE)--Dec. 18, 2017-- Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, and Sanofi Genzyme, the specialty care global business unit of Sanofi, announced today the submission of a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for patisiran, an investigational RNAi therapeutic targeting transthyretin (TTR) for the treatment of adults with hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis). Patisiran was previously granted accelerated assessment by the EMA, potentially reducing the Agency's evaluation time from 210 to 150 days.

"The MAA submission for patisiran represents another important milestone for Alnylam and a critical step toward bringing RNAi therapeutics to people living with hATTR amyloidosis," said Eric Green, Vice President and General Manager of the TTR program at Alnylam. "Based on the results of the APOLLO study, we believe patisiran has the potential to become the standard of care for the treatment of hATTR amyloidosis. We look forward to working with the EMA and the Committee for Medicinal Products for Human Use (CHMP) during the review process."

"People with hATTR amyloidosis have limited treatment options," said Rand Sutherland, M.D., Therapeutic Area Head, Rare Diseases Development at Sanofi. "With this MAA submission, we are one step closer to making patisiran available in Europe and executing on our shared vision to bring this RNAi treatment to patients globally."

Alnylam announced completion of the submission of a New Drug Application with the U.S. Food and Drug Administration on December 12, 2017. Sanofi Genzyme is currently preparing regulatory filings for patisiran in Japan, Brazil and other countries, with submissions expected to begin in the first half of 2018. Pending regulatory approvals, Alnylam will commercialize patisiran in the U.S., Canada and Western Europe, with Sanofi Genzyme commercializing the product in the rest of the world, including certain Central and Eastern European countries of the European Union.

About Patisiran
Patisiran is an investigational intravenously administered RNAi therapeutic targeting transthyretin (TTR) in development for the treatment of hereditary ATTR amyloidosis. It is designed to target and silence specific messenger RNA, potentially blocking the production of TTR protein before it is made. This may help to enable the clearance of TTR amyloid deposits in peripheral tissues and potentially restore function to these tissues. The safety and efficacy of patisiran have not been evaluated by the U.S. Food and Drug Administration or any other health authority.

About APOLLO Phase 3 Study
The APOLLO Phase 3 study (N=225) was a randomized, double-blind, placebo-controlled, global study designed to evaluate the efficacy and safety of patisiran in hATTR amyloidosis patients with polyneuropathy. The study was completed in August 2017 and detailed study results were presented at the 1st European ATTR Amyloidosis Meeting for Patients and Doctors on November 2, 2017. All patients completing the APOLLO Phase 3 study are eligible to screen for the Global OLE study, in which they have the opportunity to receive patisiran on an ongoing basis.

About hATTR amyloidosis
Hereditary transthyretin (TTR)-mediated (hATTR) amyloidosis is an inherited, progressively debilitating, and often fatal disease caused by mutations in the TTR gene. TTR protein is produced primarily in the liver and is normally a carrier of vitamin A. Mutations in TTR cause abnormal amyloid proteins to accumulate and damage body organs and tissue, such as the peripheral nerves and heart, resulting in intractable peripheral sensory neuropathy, autonomic neuropathy, and/or cardiomyopathy. hATTR amyloidosis represents a major unmet medical need with significant morbidity and mortality, affecting approximately 50,000 people worldwide. hATTR amyloidosis patients have a life expectancy of 2.5 to 15 years from symptom onset, and the only approved treatment options are liver transplantation for early stage disease and tafamidis (approved in Europe, Japan and certain countries in Latin America, specific indication varies by region). There is a significant need for novel therapeutics to help treat patients with hATTR amyloidosis.

About LNP Technology
Alnylam has licenses to Arbutus Biopharma LNP intellectual property for use in RNAi therapeutic products using LNP technology.

About RNAi
RNAi (RNA interference) is a natural cellular process of gene silencing that represents one of the most promising and rapidly advancing frontiers in biology and drug development today. Its discovery has been heralded as "a major scientific breakthrough that happens once every decade or so," and was recognized with the award of the 2006 Nobel Prize for Physiology or Medicine. By harnessing the natural biological process of RNAi occurring in our cells, 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, function upstream of today's medicines by potently silencing messenger RNA (mRNA) – the genetic precursors – that encode for disease-causing proteins, thus preventing them from being made. This is a revolutionary approach with the potential to transform the care of patients with genetic and other diseases.

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 the case of patisiran, Alnylam will advance the product in the United States, Canada and Western Europe, while Sanofi Genzyme will advance the product in the rest of the world.

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 people afflicted with rare genetic, cardio-metabolic, and hepatic infectious diseases. Based on Nobel Prize-winning science, RNAi therapeutics represent a powerful, clinically validated approach for the treatment of a wide range of severe and 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 four product candidates that are in

late-stage development. 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 to address the needs of patients who have limited or inadequate treatment options. Alnylam employs over 600 people in the U.S. and Europe and is headquartered in Cambridge, MA. For more information about our people, science and pipeline, please visit www.alnylam.com and engage with us on Twitter at @Alnylam or on LinkedIn.

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.

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.

Sanofi, Empowering Life.

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 complete results from its APOLLO Phase 3 clinical trial for patisiran and the potential implications of such results for patients, its plans for and the expected timing of regulatory filings seeking approval for patisiran from regulatory authorities in the United States, Europe and ROW countries, its expectations regarding the potential for patisiran to improve the lives of hATTR amyloidosis patients with polyneuropathy and their families, its plans for the commercialization of patisiran if approved by regulatory authorities, and expectations regarding 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.

Patisiran has not 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 this investigational therapeutic.

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 regarding the potential marketing approvals for the product. Forward-looking statements are generally identified by the words “expects”, “anticipates”, “believes”, “intends”, “estimates”, “plans”, “will be” 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, including future clinical data relating to the product ,decisions by regulatory authorities, such as the FDA or the EMA, regarding whether and when to approve the product as well as their decisions regarding labeling and other matters that could affect the availability or commercial potential of the product, the absence of guarantee that the product if approved will be commercially successful, , risks associated with intellectual property, future litigation, the future approval and commercial success of therapeutic alternatives, and volatile economic conditions, as well as those risks 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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