Alnylam Announces EMA Acceptance of Marketing Authorisation Application (MAA) for Patisiran for the Treatment of Hereditary ATTR (hATTR) Amyloidosis

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– EMA Will Evaluate MAA Under Accelerated Assessment –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 25, 2018-- Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, announced today that the European Medicines Agency (EMA) has accepted the Marketing Authorisation Application (MAA) and initiated its review for patisiran, an investigational RNAi therapeutic targeting transthyretin (TTR) for the treatment of hereditary ATTR (hATTR) amyloidosis. The filing of the MAA was previously announced on December 18, 2017, and patisiran was previously granted accelerated assessment by the EMA, potentially reducing the Agency's evaluation time from 210 to 150 days.

"By evaluating the patisiran application under accelerated assessment, the EMA acknowledges the urgent need for a new treatment approach to address the debilitating and devastating effects of hATTR amyloidosis," said Eric Green, Vice President and General Manager of the TTR program at Alnylam. "We plan to work closely with the EMA and Committee for Medicinal Products for Human Use (CHMP) toward the goal of bringing patisiran to patients with hATTR amyloidosis in the EU as quickly as possible."

In addition, Alnylam announced today that the UK Medicines and Healthcare Products Regulatory Agency (MHRA) has granted designation of patisiran as a Promising Innovative Medicine (PIM), supportive of its consideration for entry into the UK's Early Access to Medicines Scheme (EAMS). EAMS aims to give patients with life-threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization when there is a clear unmet medical need.

Alnylam announced completion of the submission of a New Drug Application with the U.S. Food and Drug Administration (FDA) on December 12, 2017. In the U.S., patisiran has Fast Track Designation, Breakthrough Therapy Designation, and an expanded Orphan Drug Designation for ATTR amyloidosis from the FDA.

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 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 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

On January 7, 2018, Alnylam and Sanofi announced a restructuring of their RNAi therapeutics alliance. As part of this restructuring, Alnylam will obtain global development and commercialization rights to patisiran and Sanofi Genzyme will be eligible to receive royalties on sales of patisiran in territories outside the United States, Canada and Western Europe. Sanofi intends to substantially complete the transition of its patisiran activities in regions outside the United States, Canada, and Western Europe, consistent with the original scope of its license rights to patisiran, by mid-2018. The transaction is subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act.

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 700 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.

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 patisiran, its expectations regarding the review, potential regulatory approval and commercial launch of patisiran in the United States and Europe, 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 its safety or effectiveness.

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