Alnylam Receives Orphan Drug Designation from the United States Food & Drug Administration for ALN-TTRsc02, a Subcutaneously Delivered Investigational RNAi Therapeutic for the Treatment of Transthyretin-Mediated Amyloidosis

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 4, 2018-- Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, announced today that the United States Food and Drug Administration (FDA) has granted Orphan Drug Designation to ALN-TTRsc02, an investigational RNAi therapeutic for the treatment of transthyretin-mediated amyloidosis. ALN-TTRsc02 has the potential to be a once-quarterly, low volume, subcutaneously administered RNAi medication in the management of this serious disease.

"We are very pleased to have received Orphan Drug Designation from the FDA for ALN-TTRsc02, which we believe has the potential to become a meaningful treatment option for people living with ATTR amyloidosis, a progressive, debilitating, and often fatal disease," said Rena Denoncourt, Program Leader, ALN-TTRsc02 Program at Alnylam. "We look forward to the continued development of this investigational therapeutic, including advancing the program into Phase 3 later this year."

The FDA Orphan Drug Designation Program provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S.

The European Commission also recently issued the decision to adopt the opinion of the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) and designate ALN-TTRsc02 as an orphan medicinal product in the European Union for the treatment of transthyretin-mediated amyloidosis.

About ALN-TTRsc02

ALN-TTRsc02 is an investigational, subcutaneously administered RNAi therapeutic targeting transthyretin (TTR) in development for the treatment of TTR-mediated (ATTR) amyloidosis. It is designed to target and silence specific messenger RNA, potentially blocking the production of wild-type and mutant TTR protein before it is made. This may help to reduce the deposition and facilitate the clearance of TTR amyloid deposits in peripheral tissues and potentially restore function to these tissues. The safety and efficacy of ALN-TTRsc02 have not been evaluated by the U.S. Food and Drug Administration, European Medicines Agency or any other health authority.

About ATTR amyloidosis

Transthyretin (TTR)-mediated (ATTR) amyloidosis is a rare, progressively debilitating, and often fatal disease caused by misfolded TTR proteins that accumulate as amyloid deposits in multiple tissues including the nerves, heart, and GI tract. TTR protein is primarily produced in the liver and is normally a carrier of vitamin A. Hereditary ATTR (hATTR) amyloidosis is an inherited, progressive disease that occurs when mutations in the TTR gene cause abnormal amyloid proteins to accumulate and damage body organs and tissues, such as the peripheral nerves and heart, resulting in intractable peripheral sensory neuropathy, autonomic neuropathy, and/or cardiomyopathy, as well as other disease manifestations. hATTR amyloidosis represents a major unmet medical need with significant morbidity and mortality, affecting approximately 50,000 people worldwide. The median survival is 4.7 years following diagnosis, with a reduced survival (3.4 years) for patients presenting with cardiomyopathy. Wild-type ATTR (wtATTR) amyloidosis is a nonhereditary, progressive disease of undefined etiology that occurs when misfolded TTR proteins accumulate as amyloid deposits in multiple organs, including the heart, resulting predominantly in cardiomyopathy, leading to heart failure and mortality within 2 to 6 years. Prevalence of wtATTR amyloidosis is uncertain, however estimates suggest fewer than 200,000 patients across the U.S. and Europe.

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.

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 800 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, its expectations as to the prospects of ALN-TTRsc02, including its potential to be a once-quarterly, low volume, subcutaneously administered RNAi medication, the potential patient population who may benefit from ALN-TTRsc02, and the expected timing for initiation of the Phase 3 program for ALN-TTRsc02, as well as 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.

ALN-TTRsc02 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.

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