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Alnylam Pharmaceuticals Receives Priority Review in Canada for Patisiran, an Investigational RNAi Therapeutic for the Treatment of Hereditary Transthyretin-mediated Amyloidosis (hATTR amyloidosis) with Polyneuropathy

– Health Canada Grants Priority Review to promising medicines intended for the treatment of serious, debilitating or life-threatening conditions –

TORONTO, Canada, Sept 24th, 2018 – Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNA interference (RNAi) therapeutics company, announced today that Health Canada has granted Priority Review status to patisiran. Priority review is granted to regulatory filings in Canada intended for new potentially life-saving treatments where there is no existing medicine on the Canadian market or where the new medicine represents a significant improvement in the benefit/risk profile over existing products. Currently there are no approved treatments for patients with hATTR amyloidosis in Canada.

Many serious, chronic and life-threatening diseases, such as hATTR amyloidosis, are caused by a fault or mutation that interferes with the way the body manufactures proteins. RNAi is a completely new approach to the treatment of these diseases; targeting the faulty protein that is causing the disease rather than treating the symptoms. The discovery of RNAi was awarded the Nobel Prize in Medicine in 2006. RNAi therapeutics are an entirely new class of medicines.

"We are delighted to have received Priority Review status for patisiran and look forward to continuing to work closely with Health Canada during the review process," said Jeff Miller, Country Manager, Alnylam Canada. "The Priority Review underscores the urgent need for an approved treatment for Canadian patients with hATTR amyloidosis, an aggressive, rapidly progressive, debilitating and fatal disease."

The regulatory submission in Canada for the treatment of patients with hATTR amyloidosis will be based on data from the APOLLO Phase 3 study. The randomized, double-blind, placebo-controlled, global Phase 3 is the largest-ever study conducted in hATTR amyloidosis patients with polyneuropathy, and enrolled patients in Canada. The results were published in the July 5, 2018, issue of The New England Journal of Medicine.

"In Canada, patients diagnosed with hATTR amyloidosis, and their families, face an uncertain future and poor prognosis, with no treatments approved to halt or reverse the progression of the disease. In the APOLLO study, patisiran-treated patients saw an improvement in their polyneuropathy symptoms, quality of life and ability to undertake day-to-day tasks, compared to those treated with placebo. Priority review designation for patisiran is an excellent step here in Canada, and I am looking forward to the outcome of the regulatory process", said Dr. Vera Bril, Professor of Medicine at the University of Toronto, Director of Neurology at University Health Network and Mount Sinai Hospital and the Krembil Family Chair in Neurology.

"We are delighted that Health Canada has granted Priority Review status to patisiran, which hopefully means Canadians with hATTR amyloidosis will soon have access to this important therapy. We are optimistic that this signals the beginning of a brighter future for patients in Canada living with this devastating, fatal disease that can affect families over generations," said Durhane Wong-Rieger, President and CEO, Canadian Organization for Rare Disorders (CORD).

About Patisiran

Patisiran, based on Nobel Prize-winning science, is an intravenously administered RNAi therapeutic targeting transthyretin (TTR) 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. Patisiran blocks the production of transthyretin in the liver, reducing its accumulation in the body's tissues in order to halt or slow down the progression of the disease. In August 2018, patisiran received U.S. Food and Drug Administration (FDA) approval for the treatment of the polyneuropathy of hATTR amyloidosis in adults, as well as European Medicines Agency marketing authorization for the treatment of hATTR amyloidosis in adults with Stage 1 or Stage 2 polyneuropathy. In Canada, the safety and efficacy of patisiran are still under review and market authorization has not yet been granted.

About hATTR amyloidosis

Hereditary transthyretin (TTR)-mediated amyloidosis (hATTR amyloidosis) is an inherited, progressively debilitating, and often fatal disease caused by mutations in the TTR gene. TTR protein is primarily produced in the liver and is normally a carrier of vitamin A. Mutations in the TTR gene 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, 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. In Canada, there are no approved treatment options that can modify the course of the disease and there remains a pressing need for novel medicines to help treat these patients.

About RNAi
RNAi (RNA interference) is a natural cellular process of gene silencing that represents one of the most promising and rapidly advancing frontier 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 continue to transform the care of patients with genetic and other diseases.

About Alnylam
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 worldwide and is headquartered in Cambridge, MA.

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 data supporting the Canadian regulatory submission and the potential implications of such data for patients, plans for regulatory filings in other markets and expectations regarding the company's "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, successfully launching, marketing and selling its approved products globally, Alnylam’s ability to successfully expand the indication for patisiran in the future, 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.

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