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Alnylam Announces Alignment on Value-Based Agreements with Leading Health Insurers and Launches Comprehensive Patient Support Services for ONPATTRO™ (patisiran)

– Structure of Value-Based Arrangements Agreed in Principle with Harvard Pilgrim Health Care and Other Major Health Insurers –

– Alnylam Assist™ Program Designed to Facilitate Access to ONPATTRO and Provide Dedicated Patient Support –

CAMBRIDGE, Mass., August 10, 2018 – Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, today announced it has agreed on the structure and key terms of value-based agreements (VBA) with leading health insurers for ONPATTRO™ (patisiran) lipid complex injection, a first-of-its-kind RNA interference (RNAi) therapeutic for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults. ONPATTRO, approved today by the U.S. Food and Drug Administration (FDA), is the first FDA-approved treatment available in the United States for this indication. ONPATTRO was shown to improve polyneuropathy, with reversal of neuropathy impairment in a majority of patients, as measured by change from baseline in the modified Neuropathy Impairment Score +7 (mNIS+7) primary endpoint in the APOLLO pivotal study. In the largest controlled study of hATTR amyloidosis patients with polyneuropathy, ONPATTRO was also shown to improve quality of life, as measured by the Norfolk Quality of Life-Diabetic Neuropathy (QoL-DN) patient-reported assessment, reduce autonomic symptoms, and improve activities of daily living.

"While preparing for approval and launch, we knew that our more than 15-year effort to advance RNAi therapeutics would only be successful if ONPATTRO gets to the patients who need it," said Barry Greene, President of Alnylam. "Our pursuit of value-based agreements with insurance providers and our Alnylam Assist™ program demonstrate how we intend to operate as a commercial company, consistent with our Patient Access Philosophy. We believe these initiatives are the right things to do for patients and will deliver value to the healthcare system."

Alnylam is actively discussing VBAs with leading commercial insurers, and has reached agreement in principle on the structure of value-based agreements with Harvard Pilgrim Health Care and other major health insurers, with many ongoing discussions. Based on MMIT, a

leading medical policy reporting agency, these engaged payers cover approximately 76 percent of commercial medical lives in the U.S. The goal of these agreements is to ensure that Alnylam is paid based on the ability of ONPATTRO to deliver outcomes in the real world setting comparable to those demonstrated in clinical trials. Partnering with payers on these agreements is intended to provide more certainty to them for their investment, and help accelerate coverage decisions for patients. The agreements are structured to link ONPATTRO's performance in real-world use to financial terms.

"Alnylam's proactive approach to working closely with Harvard Pilgrim far ahead of the drug approval date to develop a value-based agreement highlights its commitment to patients, patient access, and delivering meaningful outcomes to the healthcare system," said Michael Sherman, M.D., M.B.A., Chief Medical Officer of Harvard Pilgrim Health Care. "By virtue of linking level of reimbursement to meaningful patient outcomes, this agreement will help us meet the needs of hATTR amyloidosis patients by supporting our efforts to balance access and affordability."

The VBAs build upon Alnylam's Patient Access Philosophy announced last year, which focuses the Company's commercial objectives on being proactive about patient access while delivering value to patients, physicians, and insurers. This Philosophy commits Alnylam to act with urgency for patients, pursue value-based agreements and to not increase the annualized price of its medicines above the consumer price index (CPI-U) unless valuable new innovation has been achieved.

"Express Scripts is enthusiastic about this approval as it represents a new therapeutic technology that should improve the lives of patients. We applaud Alnylam for taking a responsible approach to pricing and patient access in the rare disease space, an increasingly complicated challenge," said Steve Miller, M.D., Chief Medical Officer, Express Scripts. "We want to work together toward a common goal of ensuring broad, affordable access to important medicines for rare conditions."

Other insurers have also recognized Alnylam's approach to delivering good value through reimbursement design:

"Alnylam's approach is to start with a clinical package that provides real benefit to patients and then ensure value is delivered in the real world over time. It's about reducing uncertainty and knowing that you're paying for the outcomes you expect," said Jim Clement, Executive Director, Value Based Care and Supply Chain Management, Aetna Pharmacy Management. "I congratulate Alnylam for establishing the Company as a proactive, innovative and influential leader in the industry, and am looking forward to being a part of this Alnylam initiative."

Alnylam Assist™

Today, Alnylam also launched Alnylam Assist, a comprehensive support services program to help patients. Alnylam Assist will offer a wide range of personalized services that include access to in-house Case Managers who will assist with verification of insurance benefits and financial support for eligible patients, and field-based Patient Education Liaisons who will offer patients education on hATTR amyloidosis.

Physicians and patients can learn more about Alnylam's comprehensive patient services by visiting AlnylamAssist.com or calling 1-833-256-2748.

Speeding Availability & Accurate Diagnosis
Given the rapid and debilitating progression of hATTR amyloidosis, Alnylam is working to expedite the availability of ONPATTRO to patients whose physicians have prescribed it so that they can initiate treatment as quickly as possible. Alnylam is partnering closely with a network of specialty pharmacies and distributors to make ONPATTRO commercially available within 48 hours of FDA approval. Alnylam continues to offer its third-party genetic testing service in the U.S. and Canada, called Alnylam Act™, which is provided at no charge to patients and their physicians and aims to reduce the time to accurate diagnoses.

Visit ONPATTRO.com for more information, including full prescribing information.

IMPORTANT SAFETY INFORMATION

Infusion-Related Reactions
Infusion-related reactions (IRRs) have been observed in patients treated with ONPATTRO. In a controlled clinical study, 19% of ONPATTRO-treated patients experienced IRRs, compared to 9% of placebo-treated patients. The most common symptoms of IRRs with ONPATTRO were flushing, back pain, nausea, abdominal pain, dyspnea, and headache.

To reduce the risk of IRRs, patients should receive premedication with a corticosteroid, acetaminophen, and antihistamines (H1 and H2 blockers) at least 60 minutes prior to ONPATTRO infusion. Monitor patients during the infusion for signs and symptoms of IRRs. If an IRR occurs, consider slowing or interrupting the infusion and instituting medical management as clinically indicated. If the infusion is interrupted, consider resuming at a slower infusion rate only if symptoms have resolved. In the case of a serious or life-threatening IRR, the infusion should be discontinued and not resumed.

Reduced Serum Vitamin A Levels and Recommended Supplementation
ONPATTRO treatment leads to a decrease in serum vitamin A levels. Supplementation at the recommended daily allowance (RDA) of vitamin A is advised for patients taking ONPATTRO. Higher doses than the RDA should not be given to try to achieve normal serum vitamin A levels during treatment with ONPATTRO, as serum levels do not reflect the total vitamin A in the body.

Patients should be referred to an ophthalmologist if they develop ocular symptoms suggestive of vitamin A deficiency (e.g. night blindness).

Adverse Reactions
The most common adverse reactions that occurred in patients treated with ONPATTRO were upper respiratory tract infections (29%) and infusion related reactions (19%).
For additional information about ONPATTRO, please see the full Prescribing Information.

About ONPATTRO™ (patisiran) lipid complex injection
ONPATTRO was approved by the U.S. Food and Drug Administration (FDA) for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults. ONPATTRO is the first and only RNA interference (RNAi) therapeutic approved by the FDA for this indication. ONPATTRO utilizes a novel approach to target and reduce production of the TTR protein in the liver via the RNAi pathway. Reducing the TTR protein leads to a reduction in the amyloid deposits that accumulate in tissues. ONPATTRO is administered through intravenous (IV) infusion once every 3 weeks following required premedication and the dose is based on actual body weight. Home infusion may be an option for some patients after an evaluation and recommendation by the treating physician, and may not be covered by all insurance plans. Regardless of the setting, ONPATTRO infusions should be performed by a healthcare professional. For more information about ONPATTRO, visit ONPATTRO.com.

About hATTR Amyloidosis
Hereditary transthyretin (TTR)-mediated amyloidosis (hATTR) 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. The median survival is 4.7 years following diagnosis. Until now, people living with hATTR amyloidosis in the U.S. had no FDA-approved treatment options.

Alnylam Assist™
As part of Alnylam's commitment to making therapies available to those who may benefit from them, Alnylam Assist will offer a wide range of services to guide patients through treatment with ONPATTRO, including financial assistance options for eligible patients, benefit verification and claims support, and ordering assistance and facilitation of delivery via specialty distributor or specialty pharmacy. Patients will have access to dedicated Case Managers who can provide personalized support throughout the treatment process and Patient Education Liaisons to help patients gain a better understanding of the disease. Visit AlnylamAssist.com for more information.

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. RNAi therapeutics are a new class of medicines that harness the natural biological process of RNAi. 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 in developing medicines to improve 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 improve 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. ONPATTRO, available in the U.S. for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults, is Alnylam's first U.S. FDA-approved RNAi therapeutic. Alnylam has a deep pipeline of investigational medicines, including three 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. 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 approval of ONPATTRO™ (patisiran) lipid complex injection, including the approved indication, and the implications of such approval for patients, the results from its APOLLO Phase 3 clinical trial for patisiran, its expectations concerning when ONPATTRO will be available for shipment to healthcare providers in the U.S., its plan to offer comprehensive support services for people prescribed ONPATTRO through Alnylam Assist™, the expected timing for additional regulatory filings for approval in global markets, its expectations regarding the potential for patisiran to improve the lives of hATTR amyloidosis patients with polyneuropathy and their families, its plans to work with the FDA to expand the indication for ONPATTRO in the future, 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, successfully launching, marketing and selling its approved products globally, Alnylam's ability to successfully expand the indication for ONPATTRO 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.