Alnylam Pharmaceuticals and GENESIS Pharma Partner to Commercialize ONPATTRO® (patisiran) in South East Europe

July 30, 2019

-- ***Partnership with GENESIS Pharmamarks an important step in the global commercial expansion of ONPATTRO, the first-in-class 'gene-silencing' RNAi therapeutic*** --

ZUG, Switzerland, and Nicosia, Cyprus, 30 July, 2019 - Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company and GENESIS Pharma, a leading regional biopharma company operating in the broader region of South East Europe, announced today an exclusive agreement to commercialize ONPATTRO®, a first-in-class RNAi therapeutic, in 12 countries: Greece, Cyprus, Bulgaria, Romania, Slovenia, Croatia, Serbia, Bosnia and Herzegovina, Albania, Republic of North Macedonia, Montenegro as well as Malta.

Our partnership with GENESIS Pharma enables us to extend access to ONPATTRO to patients suffering from hereditary ATTR (hATTR) amyloidosis in areas of Europe where we currently don't have a presence," said Theresa Heggie, SVP and Head of Europe, Middle East and Africa, and Canada, Alnylam Pharmaceuticals. "We know that patients in these countries have an urgent need for new treatment options, and as the only company with an RNAi therapeutic approved in the European Union, we are delighted to partner with GENESIS Pharma to bring ONPATTRO and potential future therapies to patients in South East Europe."

Mr. Constantinos Evripides, Managing Director of GENESIS Pharma stated: "We are very happy to announce our partnership with Alnylam for the broader region of South East Europe. It is in challenging therapeutic areas, such as rare, chronic and debilitating diseases, that novel therapeutics have the most potential to bring meaningful benefits for the patients and society at large. We are committed to bringing such innovations closer to patients and the medical community in the countries that we operate, through collaborations with leading international companies such as Alnylam that transform pioneering science into medicine."
The agreement between Alnylam and GENESIS Pharma covers ONPATTRO, approved in the EU in August 2018 for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 polyneuropathy.

About ONPATTRO® (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.

Important Safety Information (ISI) for ONPATTRO

Infusion-Related Reactions

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

To reduce the risk of IRRs, patients should receive premedication with a corticosteroid, paracetamol, and antihistamines (H1 and H2 blockers) at least 60 minutes prior to patisiran 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

Patisiran treatment leads to a decrease in serum vitamin A levels. Patients receiving patisiran should take oral supplementation of approximately 2500 IU vitamin A per day to reduce the potential risk of ocular toxicity due to vitamin A deficiency. Doses higher than 2500 IU vitamin A per day should not be given to try to achieve normal serum vitamin A levels during treatment with patisiran, 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. including reduced night vision or night blindness, persistent dry eyes, eye inflammation, corneal inflammation or ulceration, corneal thickening or corneal perforation).

Adverse Reactions

The most common adverse reactions that occurred in patients treated with patisiran were peripheral oedema (30%) and infusion-related reactions (19%).

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 new class of medicines, known as RNAi therapeutics, is now a reality. 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

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, hepatic infectious, and central nervous system (CNS)/ocular 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. Alnylam's first approved RNAi therapeutic is ONPATTRO® (patisiran) available in the U.S., EU and Japan. Alnylam has a deep pipeline of investigational medicines, including five 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 1,200 people worldwide and is headquartered in Cambridge, MA.

About GENESIS Pharma

GENESIS Pharma is a regional biopharma company focused on innovative biopharmaceutical products targeting severe and rare diseases. With offices in Athens, Nicosia, Sofia, Bucharest and Zagreb, GENESIS Pharma was among the first pharmaceutical companies in Europe to specialize in the marketing, sales and distribution of biopharmaceutical products. Since its inception, GENESIS Pharma has built a strong portfolio in therapeutic areas with high unmet medical needs through long standing strategic alliances for the broader region of South East Europe with some of the leading multinational pharmaceutical companies committed to cutting edge R&D. For more information please visit www.genesispharma.com

Alnylam Forward Looking Statements

Various statements in this release concerning Alnylam's future expectations, plans and prospects, including, without limitation, Alnylam's partnership with Genesis Pharma to commercialize ONPATTRO in South East 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, 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.

Contacts:
Alnylam Pharmaceuticals, Inc.

Christine Regan Lindenboom
(Investors and Media)
+1-617-682-4340

Fiona McMillan
(Media, Europe)
+44 1628 244960

Contact:
GENESIS Pharma

Natalia Karahaliou
(Media, South Eastern Europe)
+30 2108 771605