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Alnylam Acknowledges Rare Disease Day by Highlighting Services to Aid in Rare Disease Diagnosis

- Alnylam Act™ sponsors free third-party genetic counseling and testing services for people at risk for hereditary ATTR amyloidosis (hATTR amyloidosis) -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Alnylam Pharmaceuticals, Inc. (Nasdaq:ALNY), the leading RNAi therapeutics company, today marked the 10th annual Rare Disease Day by underscoring its commitment to enabling diagnosis for people and caregivers impacted by specific rare diseases, such as hereditary ATTR amyloidosis (hATTR amyloidosis). As part of this effort, Alnylam sponsors free third-party genetic counseling and testing through Alnylam Act™ (previously known as Alnylam Assist), a program created to empower patients with the knowledge and tools to make informed decisions about their health and facilitate an early, accurate diagnosis, potentially leading to improved care. The services, currently available in the United States, are provided by independent third parties.

hATTR amyloidosis is an inherited, rapidly progressive life-threatening disease impacting 50,000[1] people worldwide. It is caused by a mutation in the transthyretin (TTR) gene that results in misfolded TTR proteins accumulating as amyloid fibrils in multiple tissues including the nerves, heart and gastrointestinal tract. The degree and severity of symptoms vary from person to person but can lead to morbidity, disability and mortality within two to 15 years of symptom onset.[1,2]

"hATTR amyloidosis is significantly under-diagnosed and often misdiagnosed because of its constellation of symptoms that may overlap with other diseases, leading many patients to experience inappropriate medical intervention, such as unnecessary medicine and surgery," said Sami L. Khella, M.D., Chief, Department of Neurology, Penn Presbyterian Medical Center and Professor of Clinical Neurology, University of Pennsylvania School of Medicine. "For my patients who have symptoms consistent with hATTR amyloidosis, Alnylam Act helps to make an accurate diagnosis."

"As of January 2017 approximately one thousand people have been tested via Alnylam Act, and nearly 16 percent of these tests were positive for a pathogenic mutation in the TTR gene, demonstrating the need to increase awareness and improve diagnosis rates," said Pritesh Gandhi, Vice President, Medical Affairs at Alnylam. "Alnylam Act is a reflection of our commitment to the hATTR amyloidosis community, and we are proud to make these complimentary third-party services available to the people at risk for, or impacted by, this progressive rare disease."

In addition to genetic testing that can be ordered by a healthcare professional, Alnylam Act allows patients and their families to connect with genetic counselors who provide education and support, serving as an advocate to help guide them through the diagnostic journey.

"It can be beneficial to meet with a genetic counselor prior to undergoing testing to understand the benefits and risks involved, including life and health insurance implications and how to work through a diagnosis," said Shawna Feely, MS, CGC, Genetic Counselor, University of Iowa. "It is our goal to help enable those at risk for hATTR amyloidosis to obtain the answers and support they need to make more informed decisions about their health and the health of their family."

For physicians interested in ordering free hATTR amyloidosis genetic testing for their patients, or for people interested in scheduling a genetic counseling session to discuss the benefits, risks and limitations of genetic testing, visit Alnylam Act.

About hATTR Amyloidosis
Hereditary ATTR amyloidosis (hATTR amyloidosis) is an inherited, rapidly progressive, life-threatening disease. hATTR amyloidosis is a multisystemic disease with a heterogeneous clinical presentation that includes sensory and motor, autonomic (e.g., diarrhea, erectile dysfunction, hypotension) and cardiac symptoms. hATTR amyloidosis can lead to significant morbidity, disability and mortality within two to 15 years. The disease continuum of hATTR amyloidosis includes patients who present with predominantly polyneuropathy symptoms, historically known as familial amyloidotic polyneuropathy (FAP), as well as patients who present with predominantly cardiomyopathy symptoms, historically known as familial amyloidotic cardiomyopathy (FAC). However, many patients suffer from both polyneuropathy and cardiomyopathy symptoms. hATTR amyloidosis represents a major unmet medical need, affecting approximately 50,000 people worldwide. The only approved treatment options for early stage disease are liver transplantation and tafamidis (approved in Europe, certain countries in Latin America and Japan, where it is approved for all stages of the disease). There is a significant need for novel therapeutics to treat patients with ATTR amyloidosis.

About RNAi
RNAi (RNA interference) is a revolution in biology, representing a breakthrough in understanding how genes are turned on and off in cells, and a completely new approach to drug discovery and development. Its discovery has been heralded as "a major scientific breakthrough that happens once every decade or so," and represents one of the most promising and rapidly advancing frontiers in biology and drug discovery today which was awarded the 2006 Nobel Prize for Physiology or Medicine. RNAi is a natural process of gene silencing that occurs in organisms ranging from plants to mammals. By harnessing the natural biological process of RNAi occurring in our cells, the creation of 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, target the cause of diseases by potently silencing specific mRNAs, thereby preventing disease-causing proteins from being made. RNAi therapeutics have the potential to treat disease and help patients in a fundamentally new way.

About Alnylam Pharmaceuticals
Alnylam is a biopharmaceutical company developing novel therapeutics based on RNA interference, or RNAi. The company is leading the translation of RNAi as a new class of innovative medicines. Alnylam's pipeline of investigational RNAi therapeutics is focused in 3 Strategic Therapeutic Areas (STArs): Genetic Medicines, with a broad pipeline of RNAi therapeutics for the treatment of rare diseases; Cardio-Metabolic Disease, with a pipeline of RNAi therapeutics toward genetically validated, liver-expressed disease targets for unmet needs in cardiovascular and metabolic diseases; and Hepatic Infectious Disease, with a pipeline of RNAi therapeutics that address the major global health challenges of hepatic infectious diseases. In early 2015, Alnylam launched its "Alnylam 2020" guidance for the advancement and commercialization of RNAi therapeutics as a whole new class of innovative medicines. Specifically, by the end of 2020, Alnylam expects to achieve a company profile with 3 marketed products, 10 RNAi therapeutic clinical programs - including 4 in late stages of development - across its 3 STArs. The company's demonstrated commitment to RNAi therapeutics has enabled it to form major alliances with leading companies including Ionis, Novartis, Roche, Takeda, Merck, Monsanto, The Medicines Company, and Sanofi Genzyme. In addition, Alnylam holds an equity position in Regulus Therapeutics Inc., a company focused on discovery, development, and commercialization of microRNA therapeutics. Alnylam scientists and collaborators have published their research on RNAi therapeutics in over 200 peer-reviewed papers, including many in the world's top scientific journals such as *Nature, Nature Medicine, Nature Biotechnology, Cell, New England Journal of Medicine,* and *The Lancet*. Founded in 2002, Alnylam maintains headquarters in Cambridge, Massachusetts. For more information about Alnylam's pipeline of investigational RNAi therapeutics, please visit www.alnylam.com.

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, its expectations regarding its STAr pipeline growth strategy, and 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 our 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 Annual Report on Form 10-K 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.

[1] Suanprasert N, et al. J Neurol Sci. 2014.

[2] Ruberg and Berk, Circulation; 126:1286-300 (2012).

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