



Alnylam Wins Prestigious Prix Galien Canada Award for Best Innovative Product with First-Ever Health Canada-Approved RNAi Therapeutic, ONPATTRO® (patisiran)

Award Recognizes ONPATTRO's Positive Impact on Patients Living with Hereditary ATTR (hATTR) Amyloidosis

Mississauga, ON, March 17, 2021 – [Alnylam Pharmaceuticals, Inc.](https://www.alnylam.com) (Nasdaq: ALNY) today announced that the Nobel Prize winning science behind ONPATTRO® (patisiran) has won the 2021 Prix Galien Canada Award for Best Innovative Product. The award, presented Wednesday March 17, recognizes a company that has developed a drug launched in the Canadian market that has made the most significant overall contribution to patient care in terms of efficacy, safety, benefits and innovation.

“Hereditary ATTR amyloidosis can be extremely debilitating and often leads to premature death, so having a treatment with the ability to potentially reverse the disease’s course of action can be life changing for patients,” said Colleen Coxson, Country General Manager of Alnylam. “ONPATTRO is our first product to launch in Canada and we believe it has marked history in several ways; including laying the foundation for a new class of medicines, RNAi therapeutics. And, this is just the beginning; there are many great innovations awaiting Alnylam in the coming years, as we look forward to continuing to harness the power of RNAi therapeutics.”

hATTR amyloidosis is a multisystemic, progressive disease caused by mutations that interfere with the way the body manufactures a specific protein formed in the liver. Known as gene silencing, RNAi therapeutics is a new approach to the treatment of the disease; targeting the faulty protein that causes the disease. ONPATTRO is the only treatment that has demonstrated improvement, relative to baseline, in both polyneuropathy and quality of life measurements in patients with hATTR amyloidosis.^{1, 2}

“ONPATTRO is an innovative, safe, and effective technology that provides treatment for a very rare disease with limited therapy and is worthy of the 2020 Prix Galien,” said Canadian Medical Hall of Fame inductee, Dr. Jean Gray at the Prix Galien Canada awards ceremony.

This is ONPATTRO’s fifth Prix Galien, having won the 2020 Best New Medicine Award in France and the 2019 and 2020 Prix Galien Award for Best Biotechnology Product in the United States, Italy and Netherlands.

About ONPATTRO (patisiran)

Patisiran is an intravenously administered RNAi therapeutic targeting transthyretin (TTR) for the treatment of hereditary ATTR amyloidosis with polyneuropathy. 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 reverse the progression of the disease.³

About hATTR amyloidosis⁴

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-motor 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.

About RNAi

RNAi (RNA interference) is a natural cellular process of gene silencing that represents a promising and rapidly advancing frontier in biology and drug development that has the potential to transform the care of patients with genetic and other diseases. It was awarded the 2006 Nobel Prize for Physiology or Medicine.

About Alnylam Pharmaceuticals

Alnylam (Nasdaq: ALNY) is leading the translation of RNA interference (RNAi) into a new class of innovative medicines with the potential to improve the lives of people afflicted with rare genetic, cardio-metabolic, hepatic infectious, and central nervous system (CNS) 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 has a deep pipeline of investigational medicines, including five product candidates that are in Phase 3 clinical trials and one in registration. 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 1200 people worldwide and is headquartered in Cambridge, MA. Alnylam Canada is headquartered in Mississauga, Ontario with established operations since June 2018.

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1 Adams et al. Patisiran, an RNAi Therapeutic, for Hereditary Transthyretin Amyloidosis. *New England Journal of Medicine*, 379:11-21, July 5, 2018.

2 Patisiran Product Monograph, Alnylam Pharmaceuticals Inc, June 7, 2019.

3 Adams et al. Patisiran, an RNAi Therapeutic, for Hereditary Transthyretin Amyloidosis. *New England Journal of Medicine*, 379:11-21, July 5, 2018.

4 The American Journal of Managed Care <https://www.ncbi.nlm.nih.gov/pubmed/28978215/> Accessed June 17, 2019.