Alnylam Corporate Fact Sheet

Harnessing a Revolution in Biology for Human Health®
Alnylam is the industry leader in the translation of RNA interference (RNAi) into a new class of innovative medicines with the potential to address the unmet needs of patients with debilitating diseases. Founded in 2002, Alnylam was built upon a bold vision of turning scientific possibility into reality, and is now advancing a robust pipeline of investigational RNAi therapeutics, with three approved products in the market.

We are dedicated to demonstrating that bold science, perseverance, and passion can come together to improve patients’ lives. Because we believe that no patient should have to wait for hope, we accept the challenges inherent in scientific discovery and the creation of a new class of medicines.

Rooted in Nobel Prize-Winning Science
Heralded as one of the most promising and rapidly advancing frontiers in biology and drug discovery, RNAi therapeutics are treating disease in an entirely new way. The discovery of RNAi was awarded the 2006 Nobel Prize for Physiology or Medicine.

We are committed to harnessing this revolution in biology and to delivering new products to patients on a sustainable basis. Alnylam scientists and collaborators have published research on RNAi therapeutics in over 250 peer-reviewed papers, including many in the world’s top scientific journals such as Nature, Nature Medicine, Nature Biotechnology, Cell, The New England Journal of Medicine and The Lancet.

Realizing the Promise of RNAi
Developing and realizing the promise of RNAi is our heritage and our future. RNAi has led to a breakthrough in understanding how genes function in cells. Through Alnylam’s efforts, RNAi has emerged as a powerful approach with the potential to transform treatment for patients with limited options.

Our RNAi therapeutics platform is the core of Alnylam’s discovery and development strategy. This platform has the potential to enable a streamlined path to market for our product candidates and fuels sustainable pipeline growth as we continue to evolve into a fully integrated biopharmaceutical company.

We believe the investigational medicines based on our RNAi therapeutics platform have the potential to advance the care of patients, with features including infrequent, low-volume, subcutaneous dosing; a potent and durable drug effect; potential for improved efficacy with sustained drug activity; and room temperature stability.

Products
Alnylam’s commercial RNAi therapeutic products are ONPATTRO® (patisiran), approved in the U.S., EU, Canada, Japan, Brazil and Switzerland, GIVLAARI® (givosiran), approved in the U.S., EU, Japan and Brazil, OXLUMO® (lumasiran), approved in the U.S., EU and Brazil, and Leqvio®* (inclisiran) approved in the U.S. and EU.

Visit ONPATTRO.com, GIVLAARI.com and OXLUMO.com for more information, including full Prescribing Information.

Patient Access Philosophy
Alnylam is committed to advancing innovative RNAi therapeutics for the treatment of patients with life-threatening diseases. For patients and society to benefit, these innovations must reach the people who need them. The overarching objective of Alnylam’s Patient Access Philosophy is to make the therapies we develop available to those who may benefit from them. Alnylam’s Patient Access Philosophy commits Alnylam to act with urgency for patients, proactively pursue value-based agreements with payers, and to not increase the annualized price of its medicines above the consumer price index (CPI-U) unless there has been valuable new innovation achieved.

Alnylam Assist®
As part of Alnylam’s commitment to making therapies available to those who may benefit from them, Alnylam Assist offers a wide range of services to guide patients through treatment with ONPATTRO, GIVLAARI and OXLUMO, including financial assistance options for eligible patients, and benefit verification and claims support. Patients will have access to dedicated Case Managers who can provide personalized support throughout the treatment process and Patient Education Liaisons who are available to help patients gain a better understanding of the disease. Visit AlnylamAssist.com for more information.

*Licensed to Novartis

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Product Pipeline

From science to medicine, bench to bedside, possibility to patient, Alnylam has advanced RNAi therapeutics from raw, groundbreaking, Nobel Prize-winning science to a powerful product engine.

Our deep pipeline of investigational RNAi therapeutics is focused in four Strategic Therapeutic Areas (STAs): Genetic Medicines, Cardio-Metabolic Disease, Infectious Disease, and CNS/Ocular Diseases. Four of our product candidates are in late-stage development: lumasiran for severe primary hyperoxaluria type 1, patisiran label expansion for ATTR amyloidosis, vutrisiran for ATTR amyloidosis, hypercholesterolemia, and fitusiran for hemophilia and other rare bleeding disorders.

For more information about our people, products, science and pipeline of investigational RNAi therapeutics, please visit alnylam.com and engage with us on Twitter at @Alnylam and on LinkedIn.