

# Anylam Corporate Fact Sheet

## Harnessing a Revolution in Biology for Human Health

Anylam 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, Anylam was built upon a bold vision of turning scientific possibility into reality, and is now advancing a robust pipeline of investigational RNAi therapeutic medicines and commercializing its first approved product in the U.S., with plans to expand to markets around the world.

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 hold the potential to treat 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. Anylam scientists and collaborators have published 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*, *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 Anylam'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 Anylam'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

In 2018, Anylam received U.S. Food and Drug Administration (FDA) approval for the first RNAi therapeutic, ONPATTRO® (patisiran) lipid complex injection for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) in adults. Visit [ONPATTRO.com](https://www.onpattro.com) for more information, including full Prescribing Information.

## Patient Access Philosophy

Anylam 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 Anylam's [Patient Access Philosophy](#) is to make the therapies we develop available to those who may benefit from them. Anylam's Patient Access Philosophy commits Anylam 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.

## Anylam Assist™

As part of Anylam's commitment to making therapies available to those who may benefit from them, Anylam Assist offers a wide range of services to guide patients through treatment with ONPATTRO, 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 [AnylamAssist.com](https://www.anylamassist.com) for more information.

## 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 pipeline of investigational RNAi therapeutics is focused in four Strategic Therapeutic Areas (STAr): Genetic Medicines, Cardio-Metabolic Disease, Hepatic Infectious Disease, and CNS/Ocular Diseases. Five of our product candidates are in late-stage development: givosiran for acute hepatic porphyria, lumasiran for primary hyperoxaluria type 1, vutrisiran for ATTR amyloidosis, inclisiran for hypercholesterolemia, and fitusiran for hemophilia and other rare bleeding disorders.

We have an extensive clinical safety database of more than 3,500 patients, with the longest duration of exposure of over five years. We currently have 10 active clinical programs.

- Genetic Medicines
- Hepatic Infectious Diseases
- Cardio-Metabolic Diseases
- CNS/Ocular Diseases

		HUMAN POC <sup>1</sup>	BREAKTHROUGH DESIGNATION	EARLY STAGE (IND or CTA Filed-Phase 2)	LATE STAGE (Phase 2-Phase 4)	REGISTRATION/ COMMERCIAL <sup>3</sup>	COMMERCIAL RIGHTS
<b>ONPATTRO®</b> (patisiran) <sup>2</sup>	Hereditary ATTR Amyloidosis					<span style="color: blue;">●</span>	Global
<b>Givosiran</b>	Acute Hepatic Porphyria					<span style="color: blue;">●</span>	Global
<b>Patisiran</b>	ATTR Amyloidosis Label Expansion				<span style="color: blue;">●</span>		Global
<b>Fitusiran</b>	Hemophilia and Rare Bleeding Disorders				<span style="color: blue;">●</span>		15-30% royalties
<b>Inclisiran</b>	Hypercholesterolemia				<span style="color: red;">●</span>		Milestones & up to 20% royalties
<b>Lumasiran</b>	Primary Hyperoxaluria Type 1				<span style="color: blue;">●</span>		Global
<b>Vutrisiran</b>	ATTR Amyloidosis				<span style="color: blue;">●</span>		Global
<b>Cemdisiran</b>	Complement-Mediated Diseases			<span style="color: blue;">●</span>			50-50
<b>Cemdisiran/ Pozelimab Combo<sup>4</sup></b>	Complement-Mediated Diseases			<span style="color: blue;">●</span>			Milestone/Royalty
<b>ALN-AAT02</b>	Alpha-1 Liver Disease			<span style="color: blue;">●</span>			Global
<b>ALN-HBV02 (VIR-2218)</b>	Hepatitis B Virus Infection			<span style="color: purple;">●</span>			50-50 option rights post-Phase 2
<b>ALN-AGT</b>	Hypertension			<span style="color: red;">●</span>			Global

<sup>1</sup> POC, proof of concept – defined as having demonstrated target gene knockdown and/or additional evidence of activity in clinical studies

<sup>2</sup> Approved in the U.S. for the polyneuropathy of hATTR amyloidosis in adults, and in the EU for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 polyneuropathy

<sup>3</sup> Includes marketing application submissions

<sup>4</sup> Cemdisiran is currently in Phase 2 development and pozelimab is currently in Phase 1 development; Alnylam and Regeneron are evaluating potential combinations of these two investigational therapeutics

As of April 2019

## Expanding Global Presence

Headquartered in Cambridge, Mass., Alnylam employs over 1,000 people worldwide and is rapidly growing globally, with additional offices in Norton, Mass., Maidenhead, U.K., Zug, Switzerland, Amsterdam, Netherlands, and Tokyo, Japan.

## Moving Toward 2020

“Alnylam 2020” marks our expected transition from a late-stage clinical development company to a multi-product, commercial-stage company with a robust and sustainable pipeline of investigational medicines – a profile that has rarely been achieved in the biopharmaceutical industry.

We are relentless in our pursuit of the development of new therapies because patients deserve discovery. Every single one of them.

<sup>1</sup> Fitusiran is in development in collaboration with Sanofi Genzyme.

<sup>2</sup> Inclisiran is in development in collaboration with The Medicines Company.

For more information about our people, products, science and pipeline of investigational RNAi therapeutics, please visit [alnylam.com](http://alnylam.com) and engage with us on **Twitter** at [@Alnylam](https://twitter.com/Alnylam) and on **LinkedIn**.