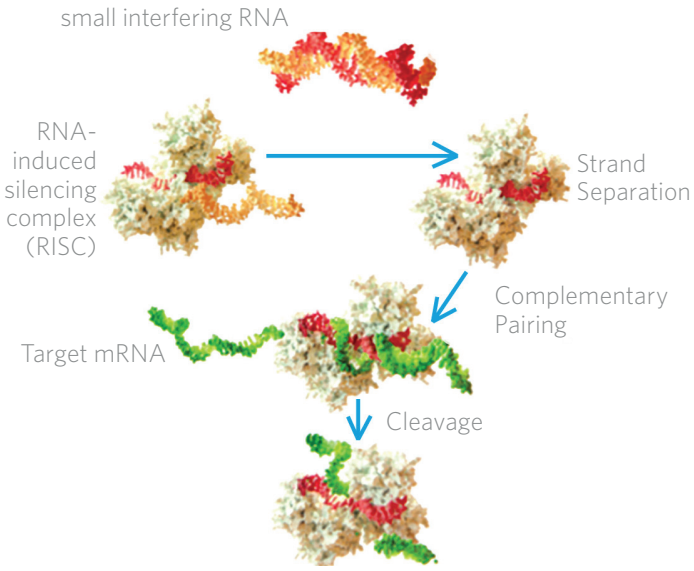
Alnylam Pharmaceuticals

Focused on Developing RNAi Therapeutics

Alnylam is a biopharmaceutical company leading the development of RNA interference (RNAi) therapeutics. The discovery of RNAi has been heralded as a major scientific breakthrough, and represents one of the most promising and rapidly advancing frontiers in biology and drug discovery today.

RNAi is a revolution in biology—a breakthrough in understanding how genes are expressed or silenced in cells. RNAi is a natural mechanism of gene silencing that occurs in organisms ranging from plants to mammals. RNAi therapeutics in development have the potential to treat diverse disease states and help patients in a fundamentally new way.



Investigational RNAi Therapeutics

Active Product Pipeline and Experience to Date

RNAi therapeutics in development by Alnylam are engineered to enable a consistent level of target knockdown. Alnylam's pipeline of investigational RNAi therapeutics is focused in four Strategic Therapeutic Areas (STArs): **Genetic Medicines, Cardio-Metabolic Disease, Hepatic Infectious Disease**, **and CNS/Ocular Diseases**. These STArs represent a range of diseases from rarest to most common globally.

PROGRAMS CURRENTLY IN CLINICAL DEVELOPMENT*
10

PEER-REVIEWED PAPERS*
>200

CLINICAL STUDIES TO DATE*
>35

LONGEST DURATION OF EXPOSURE*
>5 yrs

** As of April 2019 across all active programs. Numbers are approximate as many studies are ongoing and several are blinded.*

To learn more about Alnylam and our pipeline advancements, please visit Alnylam.com

Alnylam® PHARMACEUTICALS

Our Pipeline

Focused in 4 Strategic Therapeutic Areas (STArs):

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

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

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

[2] 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

[3] Includes marketing application submissions

[4] 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

Alnylam® PHARMACEUTICALS