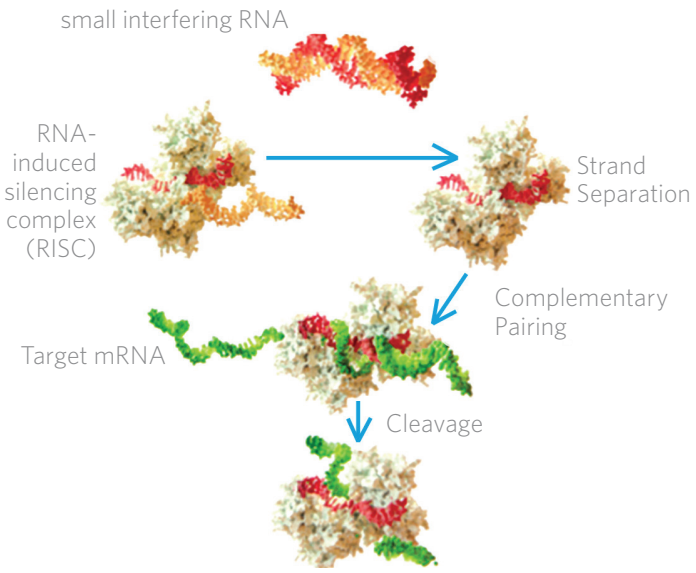
Alnylam Pharmaceuticals

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.

RNAi "Silences" Genes by Targeting specific mRNA



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 three Strategic Therapeutic Areas (STArs): **Genetic Medicines, Cardio-Metabolic Disease, and Hepatic Infectious Disease**. These STArs represent a range of diseases from rarest to most common globally.

NUMBER OF PROGRAMS IN CLINIC*
7

NUMBER OF STUDY PARTICIPANTS*
>1000

NUMBER OF CLINICAL STUDIES*
>20

LONGEST DURATION OF EXPOSURE*
>3 years

** As of November 2017 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 3 Strategic Therapeutic Areas (STArs):

- Genetic Medicines
- Cardio-Metabolic Diseases
- Hepatic Infectious Diseases

		HUMAN POC[1]	EARLY STAGE *(IND or CTA Filed-Phase 2)*	LATE STAGE *(Phase 2-Phase 3)*	REGISTRATION/ COMMERCIAL[2]
Patisiran	*Hereditary ATTR Amyloidosis*	✓			●
Fitusiran	*Hemophilia and Rare Bleeding Disorders*	✓		●	
Inclisiran	*Hypercholesterolemia*	✓		●	
Givosiran	*Acute Hepatic Porphyrias*	✓		●	
Cemdisiran	*Complement-Mediated Diseases*	✓	●		
Lumasiran	*Primary Hyperoxaluria Type 1*	✓	●		
ALN-TTRsc02	*Hereditary ATTR Amyloidosis*	✓	●		

[1]Proof of concept (POC) defined as having demonstrated target gene knockdown and/or additional evidence of activity in clinical studies
[2]Includes marketing application submissions

As of December 2017

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