

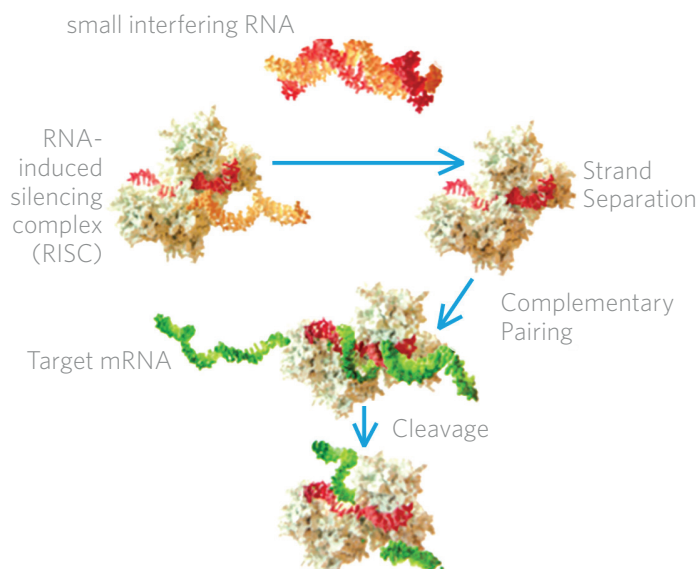
Anylam Pharmaceuticals

Focused on Developing RNAi Therapeutics

Anylam 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 Anylam are engineered to enable a consistent level of target knockdown. Anylam’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 Anylam and our pipeline advancements, please visit Anylam.com

Our Pipeline

Focused in 3 Strategic Therapeutic Areas (STAr):

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

		HUMAN POC ¹	BRAKTHROUGH DESIGNATION	EARLY STAGE <small>(IND or CTA Filed-Phase 2)</small>	LATE STAGE <small>(Phase 2-Phase 3)</small>	REGISTRATION/ COMMERCIAL ²	COMMERCIAL RIGHTS
Patisiran	<i>Hereditary ATTR Amyloidosis</i>					●	Global
Givosiran	<i>Acute Hepatic Porphyrias</i>				●		Global
Fitusiran	<i>Hemophilia and Rare Bleeding Disorders</i>				●		15-30% Royalties
Inclisiran	<i>Hypercholesterolemia</i>				●		Milestones & up to 20% Royalties
ALN-TTRsc02	<i>Hereditary ATTR Amyloidosis</i>			●			Global
Cemdisiran	<i>Complement-Mediated Diseases</i>			●			Global
Lumasiran	<i>Primary Hyperoxaluria Type 1</i>			●			Subject to partner option rights

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

²Includes marketing application submissions

As of January 2018