

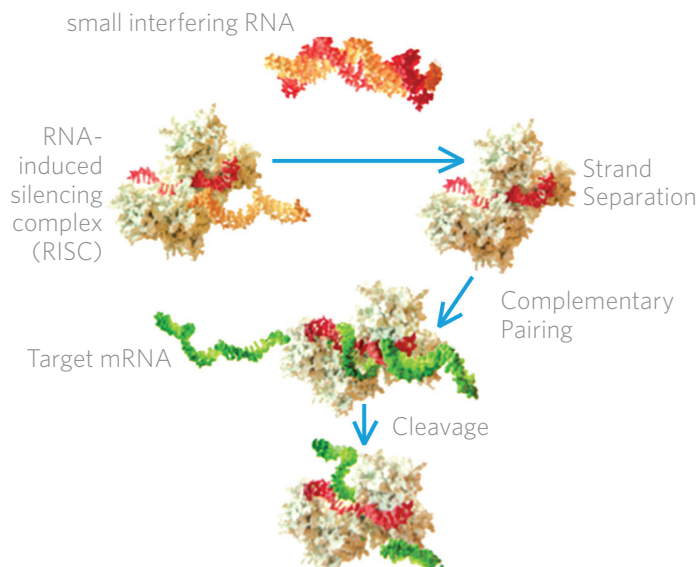
# Anylam Pharmaceuticals

*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](http://Anylam.com)

# Our Pipeline

Focused in 3 Strategic Therapeutic Areas (STAr):

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

		HUMAN POC <sup>1</sup>	BREAKTHROUGH DESIGNATION	EARLY STAGE (IND or CTA Filed-Phase 2)	LATE STAGE (Phase 2-Phase 3)	REGISTRATION/ COMMERCIAL <sup>2</sup>	COMMERCIAL RIGHTS
<b>Patisiran</b>	<i>Hereditary ATTR Amyloidosis</i>					<span style="color: #0070C0;">●</span>	Global
<b>Givosiran</b>	<i>Acute Hepatic Porphyrias</i>				<span style="color: #0070C0;">●</span>		Global
<b>Fitusiran</b>	<i>Hemophilia and Rare Bleeding Disorders</i>				<span style="color: #0070C0;">●</span>		15-30% Royalties
<b>Inclisiran</b>	<i>Hypercholesterolemia</i>				<span style="color: #800040;">●</span>		Milestones & up to 20% Royalties
<b>ALN-TTRsc02</b>	<i>Hereditary ATTR Amyloidosis</i>			<span style="color: #0070C0;">●</span>			Global
<b>Lumasiran</b>	<i>Primary Hyperoxaluria Type 1</i>			<span style="color: #0070C0;">●</span>			Global
<b>Cemdisiran</b>	<i>Complement-Mediated Diseases</i>			<span style="color: #0070C0;">●</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>Includes marketing application submissions

As of March 2018