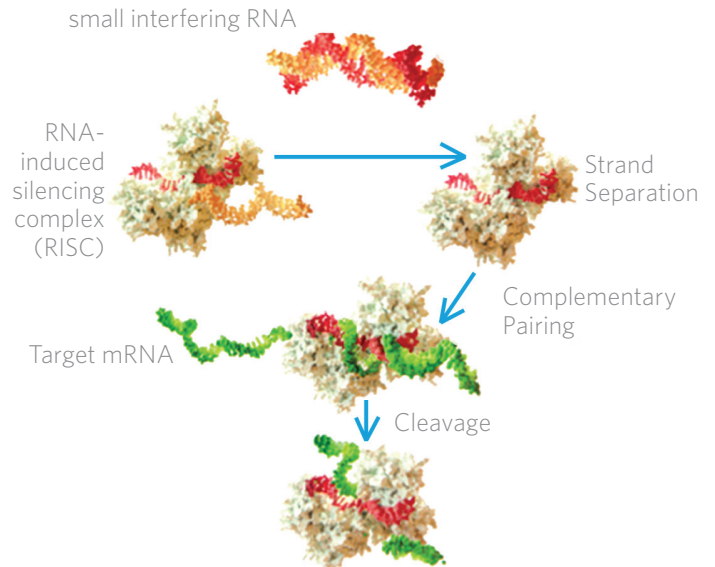


# Anylam Pharmaceuticals

*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 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 Anylam are engineered to enable a consistent level of target knockdown. Anylam's pipeline of investigational RNAi therapeutics is focused in four Strategic Therapeutic Areas (STARs): **Genetic Medicines, Cardio-Metabolic Diseases, 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\*

11

PEER-  
REVIEWED  
PAPERS\*

>250

CLINICAL  
STUDIES TO  
DATE\*

>35

LONGEST  
DURATION OF  
EXPOSURE\*

>6 yrs

\* As of March 2020 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 4 Strategic Therapeutic Areas (STAr):

- Genetic Medicines
- Cardio-Metabolic Diseases
- Hepatic Infectious 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>2</sup>	COMMERCIAL RIGHTS
<b>ONPATTRO®</b> (patisiran) <sup>3</sup>	Hereditary ATTR Amyloidosis					<span style="color: #0070C0;">●</span>	Global
<b>GIVLAARI®</b> (givosiran) <sup>4</sup>	Acute Hepatic Porphyria					<span style="color: #0070C0;">●</span>	Global
<b>Lumasiran</b>	Primary Hyperoxaluria Type 1					<span style="color: #0070C0;">●</span>	Global
<b>Inclisiran</b>	Hypercholesterolemia					<span style="color: #800040;">●</span>	Milestones & up to 20% royalties
<b>Patisiran</b>	ATTR Amyloidosis Label Expansion				<span style="color: #0070C0;">●</span>		Global
<b>Fitusiran</b>	Hemophilia and Rare Bleeding Disorders				<span style="color: #0070C0;">●</span>		15-30% royalties
<b>Vutrisiran</b>	ATTR Amyloidosis				<span style="color: #0070C0;">●</span>		Global
<b>Cemdisiran</b>	Complement-Mediated Diseases			<span style="color: #0070C0;">●</span>			50-50
<b>Cemdisiran/ Pozelimab Combo<sup>5</sup></b>	Complement-Mediated Diseases			<span style="color: #0070C0;">●</span>			Milestone/Royalty
<b>ALN-AAT02</b>	Alpha-1 Liver Disease			<span style="color: #0070C0;">●</span>			Global
<b>ALN-HBV02</b> (VIR-2218)	Hepatitis B Virus Infection			<span style="color: #4B0082;">●</span>			50-50 option rights post-Phase 2
<b>ALN-AGT</b>	Hypertension			<span style="color: #800040;">●</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

<sup>3</sup> Approved in the U.S. and Canada for the polyneuropathy of hATTR amyloidosis in adults, in the EU, Switzerland and Brazil for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 polyneuropathy, and in Japan for the treatment of transthyretin (TTR) type familial amyloidosis with polyneuropathy

<sup>4</sup> Approved in the U.S. for the treatment of adults with acute hepatic porphyria, and in the EU for the treatment of acute hepatic porphyria (AHP) in adults and adolescents over 12 years old

<sup>5</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 March 2020