

Our deep pipeline of investigational RNAi therapeutics is focused in four Strategic Therapeutic Areas (STAs): Genetic Medicines, Cardio-Metabolic Disease, Infectious Disease, and CNS/Ocular Diseases. Four of our product candidates are in late-stage development: lumasiran for severe primary hyperoxaluria type 1, patisiran label expansion for ATTR amyloidosis, vutrisiran for ATTR amyloidosis, hypercholesterolemia, and fitusiran for hemophilia and other rare bleeding disorders.

		Genetic Medicines	Cardio-Metabolic Diseases	Infectious Diseases	CNS/Ocular Diseases						
						HUMAN POC ¹	BREAKTHROUGH DESIGNATION	EARLY/MID STAGE (IND or CTA filed/Phase 2)	LATE STAGE (Phase 2/Phase 3)	REGISTRATION/COMMERCIAL ² (OLE/Phase 4/US/registries)	COMMERCIAL RIGHTS
ONPATTRO® (patisiran) ³	hATTR Amyloidosis-PN					👤	🏆			●	Global
GIVLAARI® (givosiran) ⁴	Acute Hepatic Porphyria					👤	🏆			●	Global
OXLUMO® (lumasiran) ⁵	Primary Hyperoxaluria Type 1					👤	🏆			●	Global
Leqvio® (inclisiran) ⁶	Hypercholesterolemia					👤				●	Milestones & up to 20% Royalties ⁷
AMVUTTRA® (vutrisiran) ⁸	hATTR Amyloidosis-PN					👤				●	Global
Patisiran	ATTR Amyloidosis-CM Label Expansion					👤				●	Global
Vutrisiran	ATTR Amyloidosis-CM					👤			●		Global
ALN-TTRsc04	ATTR Amyloidosis					👤		●			Global
Fitusiran	Hemophilia					👤			●		15-30% Royalties
Cemdisiran (+/- Pozelimab) ⁹	Complement-Mediated Diseases					👤			●		Global Milestone/Royalty
Belcesiran ¹⁰	Alpha-1 Liver Disease					👤		●			Ex-U.S. option post-Phase 3
ALN-HBV02 ¹¹ (VIR-2218)	Hepatitis B Virus Infection			●		👤					50-50 option post-Phase 2
Zilebesiran (ALN-AGT)	Hypertension			●		👤					Global
ALN-HSD	NASH			●		👤					Royalty
ALN-APP	Alzheimer's Disease; Cerebral Amyloid Angiopathy				●						50-50
ALN-PNP	NASH			●							50-50
ALN-KHK	Type 2 Diabetes			●							Global

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

² Includes marketing application submissions

³ Approved in the U.S. and Canada for the polyneuropathy (PN) of hATTR amyloidosis in adults, and in the EU, Japan and other countries for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 PN

⁴ Approved in the U.S., Brazil and Canada for the treatment of adults with acute hepatic porphyria (AHP), and in the EU and Japan for the treatment of AHP in adults and adolescents aged 12 years and older

⁵ Approved in the U.S. for the treatment of primary hyperoxaluria type 1 to lower urinary and plasma oxalate levels in children and adults, and in the EU and Brazil for the treatment of primary hyperoxaluria type 1 in all age groups

⁶ Novartis has obtained global rights to develop, manufacture and commercialize inclisiran

⁷ 50% of inclisiran royalty revenue from Novartis will be payable to Blackstone by Alnylam

⁸ Approved in the U.S. for the polyneuropathy (PN) of hATTR amyloidosis in adults, and in the EU and Japan for the treatment of hATTR amyloidosis with stage 1 or 2 polyneuropathy

⁹ Alnylam and Regeneron are evaluating potential combinations of the investigational therapeutics cemdisiran and pozelimab

¹⁰ Dicerna is leading and funding development of Belcesiran

¹¹ Vir is leading and funding development of ALN-HBV02

As of December 2022

Expanding Global Presence

Headquartered in Cambridge, Mass., Alnylam employs over 2,000 people worldwide and is rapidly growing globally, with additional offices in the United States and 20 other countries.

Alnylam P⁵x25

Commencing in 2022, Alnylam is executing on its “Alnylam P⁵x25” strategy to deliver transformative medicines in both rare and common diseases benefiting patients around the world through sustainable innovation and exceptional financial performance, resulting in a leading biotech profile by 2025.

We are relentless in our pursuit of the development of new therapies because patients deserve discovery. Every single one of them.

For more information about our people, products, science and pipeline of investigational RNAi therapeutics, please visit alnylam.com and engage with us on Twitter at [@Alnylam](https://twitter.com/Alnylam) and on LinkedIn.

