Alnylam Corporate Fact Sheet

Harnessing a Revolution in Biology for Human Health

Alnylam is the industry leader in the translation of RNA interference (RNAi) into a new class of innovative medicines with the potential to address the needs of patients with debilitating diseases. Founded in 2002, Alnylam was built upon a bold vision of turning scientific possibility into reality, and is now advancing a robust pipeline of investigational RNAi therapeutic medicines.

We are dedicated to demonstrating that bold science, perseverance and passion can come together to improve patients' lives. Because we believe that no patient should have to wait for hope, we accept the challenges inherent in scientific discovery and the creation of a new class of medicines.

Rooted in Nobel Prize-Winning Science

Heralded as one of the most promising and rapidly advancing frontiers in biology and drug discovery, RNAi therapeutics hold the potential to treat disease in an entirely new way. The discovery of RNAi was awarded the 2006 Nobel Prize for Physiology or Medicine.

We are committed to harnessing this revolution in biology. Alnylam scientists and collaborators have published research on RNAi therapeutics in over 200 peer-reviewed papers, including many in the world's top scientific journals such as *Nature, Nature Medicine, Nature Biotechnology, Cell, The New England Journal of Medicine* and *The Lancet.*

Realizing the Promise of RNAi

Developing and realizing the promise of RNAi is our heritage and our future. RNAi has led to a breakthrough in understanding how genes function in cells. Through Alnylam's efforts, RNAi has now emerged as a powerful, clinically-validated approach with the potential to transform treatment for patients with limited or inadequate options.

Our RNAi therapeutics platform is the core of Alnylam's discovery and development strategy. This platform has the potential to enable a streamlined path to market for our product candidates and fuels sustainable pipeline growth as we continue to evolve into a fully integrated biopharmaceutical company.

Medicines based on our RNAi therapeutics platform have the potential to overcome some of the key shortcomings of current therapies, with features including infrequent, low-volume, subcutaneous dosing; durable drug effect; potential for improved efficacy with sustained drug activity; and room temperature stability.

Product Pipeline

From science to medicine, bench to bedside, possibility to patient, Alnylam has advanced RNAi therapeutics from raw, groundbreaking, Nobel Prize-winning science to a powerful product engine. Our pipeline of investigational RNAi therapeutics is focused in four Strategic Therapeutic Areas (STArs): Genetic Medicines, Cardio-Metabolic Disease, Hepatic Infectious Disease, and CNS Diseases. Four of our product candidates are in late-stage development:

- **Patisiran: hereditary ATTR amyloidosis**
- **Givosiran: acute hepatic porphyrias**
- **Fitusiran: hemophilia and other rare bleeding disorders**
- **Inclisiran: hypercholesterolemia**

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We have an extensive clinical safety database of more than 1,000 patients, with the longest duration of exposure of up to four years. We currently have seven active clinical programs.

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

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

As of May 2018

Expanding Global Presence

Based in Cambridge, Mass., Alnylam employs more than 850 people and is rapidly growing, with additional offices in Norton, Mass., Maidenhead, U.K., Zug, Switzerland, Paris, France, and ten other countries.

Moving Toward 2020

"Alnylam 2020" marks our expected transition from a late-stage clinical development company to a multi-product, commercial-stage company with a robust and sustainable pipeline of investigational medicines – a profile that has rarely been achieved in the biopharmaceutical industry.

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, science and pipeline of investigational RNAi therapeutics, please visit **alnylam.com** and engage with us on Twitter at **@Alnylam** and on **LinkedIn**.

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