

Harnessing RNAi:

Alnylam Pharmaceuticals is conquering rare diseases one drug at a time

GOING FROM ZERO TO FIVE PRODUCTS IN FIVE YEARS

is quite a feat, but Alnylam Pharmaceuticals Inc. is aiming high for their entry into the Canadian market.

“The company was founded on the creation of therapies called RNAi—RNA interference,” explains Jeff Miller, the general manager for Alnylam Canada. “Our mission is to develop therapies for patients with diseases that were previously untreatable.”

Alnylam was one company that stuck with RNAi from the beginning. RNAi was first discovered in the 1990s, and in 2006, the Nobel Prize was awarded. The field exploded with enthusiasm, but biotech companies quickly hit a roadblock. “By around 2011, most companies had left the RNA world,” Miller explains. “But Alnylam stuck with it. The company persevered because we had a commitment to using this Nobel Prize winning technology to develop therapies for patients.”



Jeff Miller, General Manager, Alnylam Canada

FIVE PRODUCTS IN FIVE YEARS

Alnylam now has their first drug approved in Europe and the U.S., called Onpattro (Patisiran). It has been filed in Canada under priority review, and they are expecting a decision in summer 2019. Patisiran will be the first RNAi therapy to come to Canada.

Patisiran treats a disease called hereditary or hATTR amyloidosis, in which the liver produces proteins that misfold, causing amyloid fibrils. These amyloid fibrils begin to deposit throughout the body, often in the peripheral nerves and in the heart causing polyneuropathy or cardiomyopathy, as well as autonomic issues in the gastrointestinal tract. It is hard to diagnose because awareness is low, and there were only symptomatic treatments in the past.

“We hope through medical education efforts we are able to increase awareness and ultimately are able to help more patients get diagnosed sooner,” Miller says. “Our Patisiran phase three study, called APOLLO, showed unprecedented results in a disease where historically, the time from diagnosis to death was less than 5 years.”

Alnylam hopes to quickly have two follow up products in Canada. The second drug is called Givosiran, which treats Acute Hepatic Porphyria (AHP), a disease that targets predominantly younger women, causing chronic, painful and disabling attacks. In April, Alnylam reported results from the Phase 3 ENVISION study. It is expected that Givosiran will be filed for approval in Canada in the early part of 2020.

The third product in Phase 3 is called Lumasiran, which will treat Primary Hyperoxaluria Type 1 (PH1), a disease that primarily affects young children. PH1 is a disease in which excessive oxalate production results in the deposition of calcium oxalate crystals in the kidneys and urinary tract and can lead to the formation of painful and recurrent kidney stones and nephrocalcinosis.

Beyond these three important medicines, Alnylam has another two medicines in Phase 3 clinical trials and a total of 10 clinical programs in development, focused on strategic therapeutic areas including Genetic Medicines, Cardio-Metabolic Disease, Hepatic Infectious Disease, and CNS/Ocular Disease.



Steven Roblin, Medical Director, Alnylam Canada



Colleen Coxson, Head of Commercial Operations for Alnylam Canada

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BUILDING A CANADIAN PRESENCE AND BEYOND

“Having the opportunity to work for a company that is creating a completely new way to treat disease at the genetic level is a once in a lifetime opportunity,” says Steven Roblin, the Medical Director for Alnylam Canada. “It’s important for us to have a presence in Canada and be part of the Canadian community. Anything we can do to support Canadian patients is incredibly important.”

Miller was the first employee in the Canadian operation. He located offices in Mississauga, and began to build his team throughout Canada. “I’ve worked in many countries over the course of my career; the really exciting part about this particular role, in addition to the science, is coming to Canada, hiring this amazing team, building visibility for Alnylam, and serving the many patients in need of our therapies,” Miller says. “It is our goal to go from no products to hopefully five products in just a few years—that is pretty exciting!”

Globally, Alnylam is growing very quickly to serve patients in need. Currently, they have over 1,000 employees in 16 countries with a presence in the U.S. and Europe, and are rapidly expanding into Japan, Latin America, and the Middle East.

“Our core values are what make Alnylam different,” says Colleen Coxson, Head of Commercial Operations for Alnylam Canada. “Commitment to our patients is number one for us. Commitment to our people is also important—we recognize how hard people work and we support them. We are exceptionally passionate about what we’re doing.”

While Miller’s team at Alnylam is setting up their operations, they never lose sight of their ultimate goal—that patients are able to access their new and innovative treatments that could change lives for the better. “We aim to be as innovative in partnering as we have been in science,” says Miller. “We intend to deliver value to the Canadian health care system, and will be proactive with health care providers and payers to ensure that patients have access to our medicines. That is our motivation each and every day.”