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Alnylam Pharmaceuticals: Bringing groundbreaking gene silencing therapies to Ontario.

The biotech firm's expansion to Ontario coincides with FDA and EMA approval and Health Canada priority review designation for their RNA interference therapy for genetic diseases.

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The latest player in Ontario's biotech scene is bringing hope and cutting-edge new treatments for genetic diseases to Canada. Alnylam Pharmaceuticals, based in Cambridge, Massachusetts, has turned to Ontario for the next step of their international expansion, following offices opened in Europe which include the UK, Germany, France and Switzerland, and also Japan.

Alnylam's products treat genetic diseases based on RNA interference (RNAi), a discovery which was awarded the Nobel Prize in Medicine in 2006. Jeff Miller, General Manager for Alnylam Canada, explains how the science works, "RNAi, or gene silencing, targets the problem gene; the drug knocks it down and stops it from replicating. This has a huge potential benefit in treating previously untreatable genetic diseases."

Alnylam has had a big year. Their first drug, Onpattro™, has been approved by the FDA and EMA to treat hATTR polyneuropathy, a rare terminal disease that causes loss of mobility and organ failure. Before Onpattro, there was no approved treatment, and patients had an expected lifespan of four years past diagnosis. "What Onpattro has shown in modifying the disease, even reversing it in some patients, is unheard of in the history of the field," explains Miller.

16-year road to development

While Alnylam was founded in 2002, they only released their first drug this year. Miller describes the 16-year path to development as "painful at times." The first decade of development was primarily spent figuring out the drug delivery. "Now that we've figured that

out, there's an almost endless list of targets we can go after that we hope will benefit patients with diseases where there are currently limited or inadequate treatment options available," he says.

"Like any biotech company there were difficult days," Miller explains. "There was a time about a decade ago when everybody left the RNAi space, thinking it would never yield an effective therapy that could be safely delivered to human cells, but we persevered." He explains that failure wasn't an option for Alnylam founder and CEO, Dr. John Maraganore, "John has dedicated his life to developing RNA therapies, to fighting these diseases. What's amazing is his perseverance through the difficult times."

While Onpattro was in development for over a decade, Alnylam also has several other drugs in development. These include Givosiran, an RNAi therapeutic in development to treat Acute Hepatic Porphyria (AHP) and Lumasiran for Primary Hyperoxaluria Type 1. Miller explains, "Most of the drugs in our pipeline focus on rare diseases, but our development pipeline also targets more common ailments such as rare bleeding disorders and hypercholesterolemia, which affects millions of patients." Alnylam has a partnership with Sanofi on Fitusiran, an RNAi therapeutic to treat hemophilia, and with The Medicines Company on Inclisiran, an RNAi therapeutic to treat hypercholesterolemia, commonly known as high cholesterol.

Miller is confident in the future potential off RNAi therapies. Recent research has showed that RNA therapies can cross the blood-brain barrier. "That opens an opportunity to investigate its potential in diseases of the brain, such as ALS, Alzheimer's or Huntington's disease," he explains. He stresses it is early and this is only pre-clinical research, "But it still presents a huge chance for us to explore."

Community 'Incredibly welcoming and helpful'

When it came time to look for the next destination in their international expansion, the math pointed to Mississauga, Ontario. "We looked at the concentration of biotech firms like ours and saw the strong presence of pharma and biotech in Mississauga. That was the deciding factor," says Miller. He adds that as an international company, proximity to Pearson International Airport is critical. "We're also between two great academic centres, with Toronto and the excellent universities and academic community there, as well as being close to Hamilton and McMaster University," he adds.

Miller says that the strength of local universities is crucial to attract biotech businesses. "Canada has one very important element already, which is a great academic environment and reputation, with a focus on science. That's the business we're in," he says.

The Mississauga facility will house Alnylam's centralized commercialization and medical functions. As the company primarily deals with rare diseases, its medical function educates doctors about the diseases and treatment options and connects with patient networks. Clinical research will also be conducted in Canada.

Miller has lived in Europe and worked in over 20 countries, including the Greater Toronto Area, before. "As an American, I found the community to be incredibly welcoming and helpful," he says. "There's an established biotech community in the Greater Toronto Area that makes doing business easier." He adds, "There's a lot of collaboration with the academic community and scientific leadership here. It makes for great R&D opportunities."

Alnylam is currently in a phase of rapid growth as they begin to bring their long-awaited products to market. While the company has recently employed their 1000th employee, Miller estimates that number to double in the next three years. The company is also excited for the opportunity to bring RNAi treatments to the Canadian patients that will benefit from them.

"We have patients who have been on Onpattro for five years now, since the clinical trials started." Miller says. "We've seen remarkable progress in some cases. One patient, a man in our clinical trials, went back to playing the guitar. Before, he couldn't even hold a pen. Hearing about transformations such as these is what makes it all worthwhile".