BOSTON
BUSINESS JOURNAL

After fast buildup, Dimension Therapeutics aims at execution in 2016

DON SEIFFERT
Boston Business Journal

Nearly a dozen new gene therapy companies have been launched worldwide in the past few years, but none have grown faster than the one headquartered on the Charles River called Dimension Therapeutics.

Dimension (Nasdaq: DMTX) was launched in late 2013, around the same time as another Cambridge company that aims to treat (and hopefully cure) disease by inserting a new gene into cells, called Voyager Therapeutics. In a little over two years, the company has raised $160 million in two rounds of VC investment and a public offering, grown to 53 employees and opened a second facility for manufacturing in Woburn.

In this week's edition of the Business Journal, we profile CEO Annalisa Jenkins, emphasizing the 50-year-old's history as one of the first female medics ever to serve in the British Navy. But aside from Jenkin's personal background and her company's accomplishments over the past two years, it has been overshadowed by the other two locally based biotechs focused on gene therapy: bluebird bio (Nasdaq: BLUE) and Voyager (Nasdaq: VYGR).

Jenkins distinguishes her company from the others in the space in a couple of different ways. One is the method of delivering a corrected gene to a patient. Unlike bluebird, which uses a type of virus called the lentivirus, Dimension uses a much more common type called adeno-associated virus, or AAV.

JOHN SOARES

Annalisa Jenkins, CEO of Dimension Therapeutics.

But another difference between Dimension and most other gene therapy companies is its focus on diseases that involve the liver. In fact Jenkins frequently uses that fact to describe the company's place in the biotech field, comparing it to not only other gene therapy firms by to Alnylam Pharmaceuticals, which focuses on liver-based diseases using a gene-silencing approach called RNA interference.

"As Voyager is to the brain… we are going to be for the liver," she said. "And as Alnylam is RNAi for the liver, we will be AAV for the liver."

So far the company has disclosed four diseases it aims to treat: Hemophilia A and B, and two other rare conditions known as ornithine transcarbamylase deficiency and glycogen storage disease. Jenkins sees the hemophilia B program, which is now in early-stage human trials and planned to enroll up to 12 patients, as "low hanging fruit." The fact that the blood-clotting protein involved in hemophilia are produced in the liver also means it's a good fit for the company, and the abundance of research on the causes of the disease gave investors confidence in the ability of the company to treat it, she said.

"I think that sort of gave the investors the confidence that they could start to build a company around this liver-directed gene therapy, with hemophilia B being the lead program," she said.

The program aimed at hemophilia A – a more common form of the disease, but harder to treat, according to Jenkins – is partnered with German drug giant Bayer.

Beyond the four disclosed diseases, Jenkins says Dimension has narrowed down hundreds of genetically-caused, liver-directed diseases to those that are well-understood, have an associated animal model and a biomarker, and which have at least 5,000 patients worldwide. That list has about a dozen diseases on it, and Dimension is now working on seven of them.

"This year is all about executional operation," she said.