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Abeona therapeutics

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Gene Therapies for Children with Lysosomal Storage Diseases

Healthcare
Biotechnology

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Tim Miller
President & CEO

BIO: Dr. Miller has 16 years of scientific research, product development and clinical operations expertise, with a focus on transitioning novel biotherapeutics through pre-clinical phases and into Phase 1 and 2 human clinical trials. As Senior Director of Product Development at SironRX Therapeutics, he had hands-on and managerial experience evaluating complex technologies, negotiating strategic alliances and contracts with private and public organizations. He has supervisory experience in all aspects of research and development, manufacturing of biologics, and clinical program start-up from a small company perspective, with direct experience engaging Food and Drug Administration (FDA) and NIH advisory agencies on multiple Investigational New Drug (IND) submissions. Dr. Miller has led multiple teams focused on developing early-stage cardiovascular, wound healing, gene therapy and peripheral vascular disease therapies for clinical implementation. Discovery teams were structured with technical and clinical personnel to navigate concepts through product development. Management of this process included creating a portfolio management process to identify, organize, and prioritize emerging therapy opportunities. As the discoveries moved to clinic, Dr. Miller led cross-functional teams comprised of research, clinical, and regulatory personnel focused on designing and implementing clinical trials. During his career, he has contributed to multiple patent applications, managed intellectual property, and published research in several internationally recognized journals. Dr. Miller earned his PhD in Pharmacology with a focus on Gene therapy/Cystic Fibrosis from Case Western University. He also holds a B.S. in Biology and M.S in Molecular Biology from John Carroll University (Cleveland, OH).

About Abeona Therapeutics:
Abeona Therapeutics is named after a Roman Goddess who was the protector of children. Abeona was formed in early 2013 to develop therapies for rare lysosomal storage diseases and provide a unifying voice between patient advocate groups, researchers, clinicians and investors. Abeona is the result of collaborative efforts between Nationwide Children's Hospital and multiple international patient advocate groups for developing Sanfilippo therapies, including The Children's Medical Research Foundation, Inc. (USA), Team Sanfilippo (USA), Fondation Sanfilippo (Switzerland), Stop Sanfilippo (Spain), Ben's Dream: The Sanfilippo Research Foundation (USA), and the Sanfilippo Children's Research Foundation (Canada). The collaboration has helped focus parents and caregivers on a leading therapy with broad potential to provide long term benefits to children with Sanfilippo. Through this joint effort, new therapies are moving into clinical trials set to begin.

Interview conducted by:
Lynn Fosse, Senior Editor
CEOCFO Magazine

CEOCFO: Dr. Miller, Abeona is a fairly new venture for you. What is the concept?
Dr. Miller: Abeona is developing gene therapies for children with rare lysosomal storage diseases. Our first indications are Sanfilippo syndrome, Type A and Type B. These are rare genetic diseases that affect approximately one in two hundred thousand patients. There are over seven thousand rare diseases worldwide, affecting about twenty to twenty five million Americans with rare diseases. That is more than HIV or cancer combined.

CEOCFO: What attracted you personally to this concept or to working on this problem?
Dr. Miller: I've focused on transitioning novel therapies out of academic universities and hospitals into early stage clinical trials. I have been working in that space for about fifteen years and I have three children now. This disease and the preclinical research behind it was brought to me by a friend and it was very compelling. I thought that it was very viable; that it would be a great company to help start up and really help drive some of the work to get into the clinic a bit faster.

CEOCFO: What was it about this particular research that leads you to think you are on the right track?
Dr. Miller: Abeona is supported by several international foundations that have a mandate to find a cure for Sanfilippo syndrome. They have all focused their fundraising and their efforts on making this company a reality. One of the more unique things about Abeona is that this is a collaborative effort between Abeona, our partner Nationwide Children's Hospital in Columbus, Ohio and these several Sanfilippo foundations to drive this therapy forward. What was really striking about it was just how everyone had identified the science behind this particular treatment as the one that has the best shot to really help kids. There are no therapies available for Sanfilippo syndrome, and only a few in development. Our approach is less invasive and able to reach the central nervous system, a competitive advantage important for demonstrating efficacy in this disease.

CEOCFO: Would you tell us what Sanfilippo syndrome is?
Dr. Miller: Sanfilippo syndrome is a rare lysosomal storage disease affecting primarily children. Children are often diagnosed between the ages of two and six. It's a devastating neuromuscular disease–the kids often hit a developmental milestone and then spend the next number of years declining. They loose the ability to speak, the ability to walk, eventually the ability to breathe and end up passing sometimes in the second decade of life. Therefore, it takes a very large toll, both on the kids and also on the parents. It is a progressive neuromuscular disease, so there are many behavioral aspects of the disease as well as physical.

CEOCFO: What is the science that you are working on? Where are you in the process?
Dr. Miller: Our partners developed a gene therapy that in animals with Sanflippo syndrome has demonstrated remarkable success. We are right at the cusp of entering clinical trials, and have two trials slated to start; in 2014 - one each for Sanfilippo A and one for Sanfilippo B. We have entered discussions with the FDA as to what those clinical trials will look like and are in the process of submitting the regulatory paper work to really get those trials under way.

CEOCFO: How will it be delivered? Would you tell us more about that process?
Dr. Miller: The drug is a virus that has been modified to deliver the correct version of the malfunctioning gene. It is delivered as a single intravenous injection at one time. It is sort of like contracting a common cold, except that the virus is able to access more important areas of the body where the disease has its particular pathologies; such as the central nervous system. What has been demonstrated to occur in animals rather quickly over a few weeks after injection is that as the cells that pick up the drug are able to essentially remove much of the pathology that was the immediate cause of the disease..

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– Dr. Tim Miller

CEOCFO: What is the funding situation for Abeona?
Dr. Miller: Abeona recently closed our seed-financing round and is seeking funds for a series A financing round in the beginning of 2014.

CEOCFO: Is the rare disease market favorable with investors these days?
Dr. Miller: The rare disease market space has really heated up over the past two or three years. There are a few therapies available for similar diseases that are very similar to the one that we are working on. With the recent approval of the first gene therapy product in Europe, and the FDA considering approval of the first US gene therapy drug, more interest is being generated in gene therapy companies focused on rare diseases as a viable business model.

CEOCFO: What is the anticipated time table for you?
Dr. Miller: We will be continuing to raise funds for a series A into 2014, where we plan to see the initiation of two clinical trials. Those will go on through 2015. We will hopefully have the initial data from these towards the end of 2015, early 2106.

CEOCFO: It seems to be a larger group than most that have come together to focus around this particular program. Is that the case? How did the work behind the scenes put this all together so that everyone is on the same page?
Dr. Miller: About fifteen years ago a research named Dr Haiyan Fu at Nation Wide Children's Hospital focused part of her research on treatment for Sanfilippo syndrome. At that time there were very few foundations involved with trying to fund the research, because back then it was very difficult to really make find funding for a rare disease as a profitable therapy. There was an initial foundation called CureKirby by founder Sue Wilson and they provided some initial funding to Dr Fu who started more of her research into Sanfilippo. It snowballed from there, as diagnosis for Sanfilippo became more pronounced in the late 1990s and in the early 2000s more foundations started to spring up. As one of the premier Sanfilippo researchers they looked to her for potential therapies. When the technology and the therapy got to the point of getting ready to go into humans to test it, these foundations were already in the mix. They had already put some money towards the research. We are hoping to accelerate the availability of many families to access a potential therapy; these kids can get very sick very quickly, and time is of the essence.

CEOCFO: Are there international interests?
Dr. Miller: We have several international Sanfilippo foundations supporting our clinical program.

CEOCFO: From your previous ventures, what have you learned that

is most helpful as you are proceeding here?

Dr. Miller: Having a very good and communicative team around you, both on the scientific side through clinical operations and business development. It is very important to have a good communication through your scientific founders: the people involved in the company and the groups that are helping fund it. Having a very strong, existing communication base where everyone understands what the development path is has really been one of the most key things that I have found. Then having a very good team immediately around you to help execute on the deliverables.

CEOCFO: Why should Abeona stand out for investors and people in the business community?

Dr. Miller: The gene therapy we're developing has demonstrated remarkable benefits that, when taken as a whole, exceed other therapies in development. It is given as a single treatment, compared to multiple treatments per week or per month. It is a relatively non-invasive method for delivery, rather than drilling something into the back of the skull for delivery, for example, or into in the spinal chord. The actual delivery vehicle itself has already been tested and has been well-tolerated in patients in other gene therapy trials so there is a high level of safety already associated with it. Abeona will also be seeking orphan drug status, which provides market exclusivity in the US for 7 years.

CEOCFO: Any time you are investing in something in drug development it is speculative. However, your findings seem to have a really magical quality, which on one hand is great. On the other hand, does that lead to more skepticism?

Dr. Miller: That is a very interesting question. This is gene therapy. Gene therapy has been around for almost four decades. It has waxed and waned with the market. There have been some negative incidents, early in development—that really drove away investment dollars. However, over the past five to eight years we have seen a kind of resurgence on gene therapy and numerous positive results across multiple clinical trials. Therefore, between stem cells and gene therapy there is strong interest in new hopes for this technology for developing cures.

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