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Novel Drug Discovery Enabling Tissue Selective AMPK Activation

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CEOCFO: ***Dr. Batchelor, your site indicates that NovaTarg Therapeutics is bridging the innovation gap. What is the concept?***
Dr. Batchelor: We are involved in drug discovery and bridging the innovation gap is about the failure of big pharma to continue drug discovery at the pace needed to drive healthcare and their business. Therefore, there is a gap that has been opened up that will be filled by biotech companies, or small companies that are focused on drug discovery.

CEOCFO: ***What is the roll of NovaTarg in that process?***
Dr. Batchelor: We really start the earlier stages of drug discovery. We look for new opportunities for drugs that can meet unmet medical needs. Our name NovaTarg is short for Novel Targets. Therefore, it is really about new mechanisms to treat disease.

CEOCFO: ***With such a broad scope, how do you decide what to look at?***
Dr. Batchelor: I did describe that in a broad context. However, because of my experience earlier in the pharmaceutical industry I had decided that we wanted to focus on one particular mechanism, or one particular pathway. That pathway is about the activation of an enzyme called AMPK. It is a very important enzyme because it regulates energy usage in cells, and so has profound consequences for metabolic diseases and also for cancer. These are the areas that we focus on mostly.

CEOCFO: ***How does the AMPK enzyme work to regulate energy and what are you doing to activate it?***
Dr. Batchelor: What the AMPK enzyme does is it drives processes that will shift the balance towards energy conservation, rather than burning it. In terms of cancer, cell proliferation requires energy, so what AMPK does is to put the brake on cell proliferation and protein synthesis. It holds back cells from aggressively multiplying, which is what happens in the case of cancer. Therefore, it has a positive effect on cancer, but also in metabolic diseases, such as type 2 diabetes. AMPK is a key regulator of energy utilization and can be a mechanism to lower glucose levels in patients. Of course, we are also starting to understand now that there is a link between metabolic disease and cancer. They seem to go hand-in-hand as excess energy in cells is common to both processes.

CEOCFO: ***Are many people looking in this arena?***
Dr. Batchelor: AMPK activation is almost like the Holy Grail. It is an area where people have worked for many years, and there are AMPK activators known, but only one has ever been successful and gotten to the market. There have been approaches taken which can activate AMPK in cells that are in culture, but the compounds themselves do not turn out to be good enough to be effective in human beings, with one exception. That exception is the diabetes drug Metformin. What NovaTarg wants to do is discover more of those kinds of drugs so that there can be the opportunity to treat more diseases and more effectively.

CEOCFO: ***What are you working on now?***
Dr. Batchelor: The areas that we mostly work are in type 2 diabetes and another disease that we consider to be metabolic, polycystic kidney disease (PKD), which is a genetic disease. It is a fairly rare disease, but over 660,000 Americans have polycystic kidney disease. The two things that go wrong in that disease one is excess cell proliferation in the kidneys, which supports cyst formation, and then is there is excess fluid secretion that fill those cysts. The kidneys in PKD patients can grow to eight times normal size, and as they grow and become disfigured by these cysts, then their function is lost. Individuals with this disease are at high risk of end-stage renal disease, which is a life threatening condition. It turns out that AMPK regulates both cell proliferation and fluid secretion in the kidney. Therefore, the two underlying mechanisms that cause polycystic kidney disease are both regulated by this mechanism. That turns out to be a great opportunity for us. The other main area we are working on at the moment is cancer. Primarily hepatocellular carcinoma and ovarian cancer.

CEOCFO: ***With so many potential targets, how do you stay focused?***
Dr. Batchelor: That is what is interesting about this, and I hesitate to talk about the spectrum of things that we work on, but it is much less complicated than it seems. The reason for that is that we are only working on this one mechanism. Here we are looking at AMPK activators and so we are doing the chemistry to make drugs that will activate that enzyme. These compounds have this wide spectrum of potential indications. What NovaTarg does is collaborate with scientists who can help us to look at our compounds in the context of different disease settings. For example, we have a collaborator at the Kidney Institute in Kansas, Dr. Darren Wallace, and he is an expert in polycystic kidney disease. Therefore, we collaborate with him and give him compounds to test in models that show that they work in kidney disease. That is one example of how we do our business. We find interesting molecules and then we collaborate with academics.

CEOCFO: ***What gives you the confidence that you are on the right track?***
Dr. Batchelor: We are getting more and more data, to suggest we are getting very close. We have been successful with a number of grants. We have received four separate Phase 1 SBIR types of grants, which are peer reviewed grants. We are also on the verge of getting Phase 2 SBIR funding now. In addition, we can tell by the quality of molecules that we are working on that we are getting close to compounds that could be drugs. Therefore, our anticipation is that we will actually find drugs during the course of this next year. Then we will be looking at pre-clinical development and hopefully clinical development after that.

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CEOCFO: ***What is your funding situation today?***
Dr. Batchelor: The money that we received from our four Phase 1 SBIR grants, we are still using to fund our research. We have gotten about $1.5 million from NIH, so it is from Federal sources that are non-dilutive and allows us the freedom to do the research that we want to do to discover innovative drugs. There will come a point when we will need to introduce private funding as well, but we like this non-dilutive funding at this stage of our project. We have taken the grant funding approach, because it allows us to pursue research that we own ourselves outright.

CEOCFO: ***Would you tell us about your disease models and where they come from?***
Dr. Batchelor: In terms of polycystic kidney disease there are very good disease models. For example, our collaborators have access to kidneys from PKD patients, so they can grow human polycystic kidney cells in culture that we can use as model systems to test our drugs. There are also mice created with the same genetic lesion found in humans with polycystic kidney disease. Therefore, we have very good models available to us and activity of our drugs in these models gives us confidence that we are on the right track and getting closer to our goals.

CEOCFO: ***Why does NovaTarg Therapeutics standout in the healthcare space?***
Dr. Batchelor: There are a couple of reasons that NovaTarg Therapeutics should stand out. One is that we are not a common biotech company for a number of reasons, but one of the keys to it is that we are experienced pharmaceutical professionals. We have years of experience in drug discovery. I was Senior Vice President for Drug Discovery at GalxoSmithKline (GSK) in Research Triangle Park, NC. I worked with GlaxoSmithKline for 27 years and I am co-inventor of a drug called Avodart, so I have genuine experience. My colleagues in the company, Nick Livingston and Jeff Cobb are also very experienced. In truth, the only way to really know how to do drug discovery is to have spent time in the industry and then to use it in the biotech setting. I regard it as the best training that you could possibly get. Other companies may be in similar positions to us, but in general terms, academic scientists do not have the same experience that gives us the best way forward to understanding drug discovery.

It is also worth noting that we have a very strong focus. AMPK activators are what we have targeted because we know that success here will provide innovative drugs to treat both metabolic diseases and cancer.

Interview conducted by: Lynn Fosse, Senior Editor, CEOCFO Magazine

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