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NanoKor® Gene Nanotherapeutics for Treating Glioblastoma And Other Undruggable Human Diseases

Randal Goomer, PhD
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CEOCFO: ***Dr. Goomer, your website indicates that Avrygen is a leader in the exciting new field of nanomedicine. What are you doing?***
Dr. Goomer: We have pioneered the NanoKor® gene delivery platform. Using this we are able to deliver gene therapeutics such as microRNAs and siRNAs, at very high levels, to get a wide distribution of the nucleotide therapeutic payloads, with a robust expression and targetability.

CEOCFO: ***How does what you have differ from traditional medicine?***
Dr. Goomer: Traditionally, gene therapy has had a rocky road. They first were developed to be delivered by a viral method, such as adeno- associated viruses and retroviruses. There were some complications in the delivery; in particular, adeno-viruses sometimes give an inflammatory response in patients. However, they delivered the nucleotide payload at high enough levels for therapeutic effect. After that, the field went dormant when people were looking for new solutions. One of the solutions is the liposomal delivery; liposomal delivery vehicles are less toxic, however, it does not give you very high levels of efficiency of deliver of siRNAs or miRNAs. Also, using liposomes, it was not possible to achieve targetability. For example, you cannot have the payload delivered to the brain, heart or kidney. Mostly, the liposomal delivery systems are being developed and taken into the clinic for liver diseases, such as liver cancer. The NanoKor® platform is a non-liposomal, non-viral nanotherapeutic delivery system, which produces high-level delivery, while providing stability and targetability in the body. That means that the body's own immune system does not attack the NanoKor®. Therefore, they can distribute widely and because we can include certain targeting copolymers in its composition, these then go to the specific tissues, we have now done advanced pre-clinical studies to show delivery directly to the brain and to the lungs for lung cancer and lung metastasis. For brain, we have shown delivery does reduce brain tumor burden, such as in Glioblastoma and for brain metastasis, which happens either in metastatic lung cancers or during the development of advanced prostate cancers.

CEOCFO: ***What happens inside of the body? What have you figured out that others have not, so that the nanoparticles when delivered will work?***
Dr. Goomer: The NanoKor® particle itself has an organized assembled copolymer composition that encapsulates the therapeutic microRNAs, therapeutic silencerRNAs and messengerRNAs. It keeps them in a core, which is why we call it nanokor. Certain co-polymers, precluding an attack from the body's immune system, protect the internal payload-containing core. However, there are other co-polymers that we include which allow it to target specific tissues and bind to those specific cells in the tissues, such as those found in glioblastoma, the brain, lung and prostate cancer. That is a very novel approach. Scientists have thought about it, but have not been able to put it in practice, and we have with the NanoKor® platform. We have actually put it into practice and we have generated preclinical data in our model systems, where we have been able to reduce the size of the tumors, for example, the highly aggressive brain tumors. Glioblastoma patients expire, within 15 months, on average, after diagnosis. When we put the same patient derived tumors into the mice and then give the mice the siRNA or microRNA therapeutics loaded into the NanoKors®, it significantly reduced size of the glioblastoma or brain cancer in these mice.

CEOCFO: ***Is the medical community aware of what you are doing in this area? Has there been interest or is it too early?***
Dr. Goomer: It is somewhat too early. We have been fortunate enough to receive federal funding from the National Cancer Institute and National Institute of Health. In addition, we previously received funding from the Department of

Defense, such as the Army Medical Material Command, and from the Chem/Bio Program. Now we are putting the word out, approaching large Pharma companies and Venture capital groups, and, are getting ready to publish our preclinical studies.

CEOCFO: ***How have you decided what to go after first?***
Dr. Goomer: Some if it is serendipity. I am working with Dr. Liliana Soroceanu, who is a pioneer in the field of brain cancer, such as glioblastoma multiforme and with her collaboration, she produced a model of the brain cancer and then we injected the therapeutic NanoKor® into the mice. This was also awarded a Small Business Innovation Grant from the National Cancer Institute, so that allowed us to move forward and perform the studies on brain cancer. Another one is lung metastasis, there we are working with Dr. Mohammed Kashani-Sabet, and his laboratory has pioneered the models where they inject the human lung metastatic cancer cells and produce lung metastasis. We have been able to inject these mice with our therapeutic NanoKor® and have shown that we can dramatically decrease the number of cancer lesions in these mice, giving pre-clinical proof that the NanoKor® function as we have discussed.

CEOCFO: ***Are you working in an area that is getting attention from the investment community, as interest can be very cyclical?***
Dr. Goomer: That is very much the case. About five years ago as I was approaching the venture community and other large pharma companies, asking them if they were interested in this gene therapeutic, NanoKor® type platform, we were told that there was no interest. This was also partly because it was a time when the investor community was barely coming out of the great recession in 2010. Therefore, it was very difficult to launch the company and move it forward. However, we were fortunate enough to get some funding from the Army and that allowed us to develop some of these therapeutics.

"That is where the greatest excitement comes in and we believe that the next thirty years will be defined, at least in the pharma and therapeutic community, by this high level, targeted, stable NanoKor® gene therapy." - Randal Goomer, PhD

CEOCFO: ***We reach many investors as well as people in healthcare. Why pay attention to Avrygen today?***
Dr. Goomer: The most important thing is that we have created the NanoKor® platform, which is head and shoulders above any of the technologies for gene therapeutics that have been developed to date. It is unequivocally the best system for gene therapeutics for the delivery of microRNAs, silencerRNAs and messengerRNAs. Not only have we shown that we can do systemic delivery and then have demonstrated efficacy in pre-clinical models, but we have also shown that we can do this delivery by the oral route. We have produced therapeutic effect in the brain, as well as in the small intestine, after delivering by the oral route. All of these things open up a wide area of targeted gene therapeutics, which have eluded our technical community up to this point. That is where the greatest excitement comes in and we believe that the next thirty years will be defined, at least in the pharma and therapeutic community, by this high level, targeted, stable NanoKor® gene therapy.

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

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