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Microsatellite Technology for Rare Disease and Cancer Diagnostics and Drug Development

Michael "Buz" Waitzkin
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CEOCFO: ***Mr. Waitzkin, Genomeon is a microsatellite company. Would you explain microsatellites?***
Mr. Waitzkin: There are within the genome about a million brief segments of genomic data, called microsatellites or short tandem repeats. Their most common use is in forensic identification, which uses about 20 highly variable microsatellites. They are also used to diagnose certain rare diseases, such as Huntington's Disease or Fragile X.

However, the highly repetitive nature of these short sequences tends to confound current sequencing software, which either then blocks them out or inaccurately reassembles them.

Genomeon's technology is the first systematic effort to accurately read and farm all one million microsatellites to identify informative patterns of variation between healthy and diseased populations and to identify new drug targets.

CEOCFO: ***What do you understand at Genomeon that allows you to do this?***
Mr. Waitzkin: My partner, Harold "Skip" Garner, Jr., Ph.D. (CSO, Co-Founder), is a plasma physicist by training and he became involved in a genome project at the outset, when the computational challenges were new to many biologists. He collaborated with a biologist at the Salk Institute, and got one of the original genome grants. Skip became fascinated with the short tandem repeats or microsatellites, which others were dismissing as "junk DNA". However, from his perspective as a physicist he did not believe that nature creates complex systems and replicate them over millions of generations if they have no value. He spent the better part of twenty years studying microsatellites, developing a method for accurately reading them and finding hidden value within them

CEOCFO: ***Where are you today in the use of your technology? Are you commercialized or still in development?***
Mr. Waitzkin: We are still in development. Within the company, we have used the technology for a number of purposes. First, we have been able to identify patterns in DNA derived from germ line, not from tumors, to distinguish individuals who have a high likelihood of developing various kinds of cancers and other heritable diseases with a sensitivity and specificity far greater then what is available with other diagnostics. We have established proof of concept on this application and are seeking licensing relationships with other either diagnostic or pharma companies. That is one use. We also have recently developed and just filed our patent application for what we are calling a Global Microsatellite Target Enrichment Kit, which only targets microsatellites. Therefore, we are able to get a comprehensive read of virtually all of the microsatellites in the genome and we are making some very interesting discoveries. Among other things it is beginning to look like we are able to sequence much of the 1% of the genome that has not been able to be sequenced up till now.

CEOCFO: ***What is the science behind your technology?***
Mr. Waitzkin: When sequencing is done it chops up the genome into relatively short segments, and reads and reassembles them. Because the short tandem repeats or the microsatellites are not easily reassembled, they are screened out. What we do is rather than reassembling the genome in the current mode, we basically do a million small reassemblies. By looking at each of the repeats in that way, we are able to accurately sequence these microsatellites in ways that current software does not. Just to give you a sense of this, when the 1000 Genomes Project first was made public and the sequences were made available, Dr. Garner discovered that the reassembled microsatellites were only

20% accurate, because of problems with the software. However, with our proprietary algorithms, he was able to increase that accuracy up to 98%. We then created a library of about 10,000 both healthy and diseased individuals with accurately sequenced microsatellites and we could then begin making comparisons of those population groups. The simplest way to describe the capability of our technology, it is that we can differentiate populations by use of the microsatellites – healthy from diseased, drug responders from non-responders, etc. – with far greater sensitivity than other approaches

CEOCFO: ***Is the portion of the medical community that should be aware of what you are doing knowledgeable?***
Mr. Waitzkin: Not yet. There had been this common view in the scientific community that this was all junk DNA, which was an astonishing use of language. Because we did not know what it was and we did not know how to accurately read it, it was declared "junk". It would have been more humble – and more accurate - to say that we did not yet know what it is and we did not yet know how to accurately read it. It is only now that people are beginning to realize that those segments of the genome that had not previously been readable or understood might actually have value. Therefore, there is an increasing awareness of that potential, but it has been slow in coming because there were so many presumptions in the other direction and most of the scientific effort and funding was going towards trying to identify SNPs which have yielded less than had been hoped. Now attention is beginning to turn to the microsatellites as a potentially valuable area of information within the genome and we are ready for that awareness.

"Genomeon's technology is the first systematic effort to accurately read and farm all one million microsatellites to identify informative patterns of variation between healthy and diseased populations and to identify new drug targets." - Michael "Buz" Waitzkin

CEOCFO: ***Would you tell us about your partnership with Cloud Pharmaceuticals?***
Mr. Waitzkin: Edwin R. Addison Ph.D., who runs Cloud Pharmaceuticals, Inc., and is its Co-Founder, is a long time colleague of my partner Skip Garner. It is a very natural relationship between the two companies because Cloud has this wonderful technology that is able to take biomarkers and identify potential drugs that target them. What we are able to do is identify biomarkers that were not otherwise known through the use of our microsatellite technology. Therefore, it is a natural blend. For example, when we compared a population of women who had not been diagnosed with breast cancer to a population of women who were healthy, we identified 55 microsatellites, which distinguished the two populations with sensitivities and specificities in the range of 80% to 90%. Then when we looked at the location of those 55 microsatellites in the genome, only about half of them had previously been identified as potentially involved in breast cancer. Hence, each of the newly identified microsatellites in the pattern represented a potential new drug target. Importantly, about half of the remainder, while they had been previously associated with cancers, had not been identified as having been associated with breast cancer. Therefore, each of those particular microsatellites represented a new potential biomarker or drug target for breast cancer, and working with Cloud Pharmaceuticals, they can then transform those into potential drugs.

CEOCFO: ***You have a long history in biomedical research. What have you learned over time that is guiding your next steps?***
Mr. Waitzkin: One thing that I have learned is that things move a great deal slower than I would have hoped, so the first thing that I have learned is patience. We have also learned that steady progress and confidence in the technology is essential, and continuing to do research and expand the applications is essential to ultimate acceptance in the medical community.

CEOCFO: ***Do you attend many conferences and are you reaching out to the investment community?***
Mr. Waitzkin: Our approach has really been much more towards individual contacts with either potential funders or collaborators, rather than being on the lecture circuit. My partner Skip Garner has published 20 plus articles in the last 24 months in peer review journals on various aspects of our technology and over a hundred in the past two decades. So, the primary method of getting out the word is through peer review publications. We are currently in discussions with several groups about both funding and collaboration

CEOCFO: ***In the digital world, what terms would someone search for to find Genomeon?***
Mr. Waitzkin: If people were to look under microsatellites they would find us. It is an odd expression in genomics, though it is commonly accepted within the narrow community. I am very happy with the name we chose, Genomeon, as people will look for us under our name, and we use the tagline The Microsatellite Company. As more and more information comes out about the use of microsatellites and their sensitivities as diagnostics and in identifying drug targets, I think that they will find their way to us.

CEOCFO: ***Is there competition in the development of microsatellite technologies?***
Mr. Waitzkin: There is relatively little competition in the development of microsatellite technologies. We monitor that very closely. It may be because there simply is not very much information available about it. The current technologies do not accurately sequence the microsatellite regions, so if someone were to take a whole genome sequence or an exome sequence and compare the microsatellite regions in one to another, you would have 80% inaccurate information, so what you are getting is noise. Without a proprietary technology like ours that focuses exclusively on the microsatellites and accurately reads them, it is virtually impossible to conduct any effective research in the area.

CEOCFO: ***Why are you confident that people will pay attention and will embrace your technology?***
Mr. Waitzkin: I look to my partner for that confidence. He has been working on this for 20 years and he has an abiding faith that the science is absolutely rock solid. Everything that I have seen as a lay person suggests to me that the technology is there. So it is just the question of gaining acceptance from other parties. It has great scientific merit and acceptance will follow, as will commercial value.

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

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For more information visit:
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