Tom Cruise and DNA-binding Proteins

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June 10, 2010 By POZ Staff

By Ian Anderson (President, Smart + Strong)

Tim Horn, editor-in-chief of AIDSmeds, recently invited me to sit in on a meeting he was having with several folks from Sangamo BioSciences Inc. to hear about their research and development efforts.

The meeting was specifically to cover Sangamo's efforts within HIV, notably its focus on the research and development of novel DNA-binding proteins for therapeutic gene regulation and modification. The meeting had a very technical-slant, as at times I felt like I was in the final round of the National Spelling Bee.

While Tim was keeping up and blitzing them with questions, I was very appreciative of the colorful slides (like the one pictured here) used to illustrate zinc finger DNA-binding proteins (ZFPs) and ZFP transcription factors (ZFP TFs).

What I did take away from the meeting, other than a bunch of words to go look up in the dictionary, was a profound new-sense of encouragement that there was development out there with the very real potential to help people living with HIV.

Their attempts to develop a process to alter a person's DNA to curtail diseases sounds like something from a Tom Cruise movie. One effort, discussed in detail by Sangamo president and CEO Edward Lanphier, involves blocking the gene responsible for producing CCR5 receptors on CD4 cells used by HIV--a Phase I study is currently under way and another study is being planned.

The same technology is being studied in other diseases as well, including diabetic neuropathy, amyotrophic lateral sclerosis (Lou Gehrig's disease) and glioblastomas (brain cancer), to name a few.

It is surrounded by all kinds of debate on whether gene therapy is something human's should even be attempting. But the point is they are trying something to help people suffering from all kinds of afflictions.

With the recent news from Myriad and Avexa that they are shelving development of their “small molecule” HIV drugs, it was very encouraging to see a company pushing ahead in an entirely new direction. I for one can’t wait for the movie.

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