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Might I finally take part in a Huntington's disease clinical trials? An update on the latest research

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At Risk for Huntington's Disease

HD is a genetically caused brain disorder that causes uncontrollable bodily movements and robs people's ability to walk, talk, eat, and think. The final result is a slow, ugly death. Children of parents with HD have a 50-50 chance of inheriting the disease. There is no cure or treatment.

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SUNDAY, NOVEMBER 01, 2015

Might I finally take part in a Huntington's disease clinical trial? An update on the latest research

In the 20 years of my family's fight against Huntington's disease – we discovered my mom had HD the day after Christmas of 1995 – I felt this past week for the first time that I might have a chance to avoid its inevitable, mind-destroying symptoms.

On October 26, I learned about a new clinical trial aimed at rescuing brain cells from the degeneration caused by Huntington's.

Called "SIGNAL" (alternatively, VX15/2503 Treatment for Huntington's Disease), the trial will include asymptomatic carriers of the HD gene like me who are close to predicted age of onset. Made by the Rochester, NY-based biotech company Vaccinex, VX15/2503 is a monoclonal antibody, a type of molecule essential in molecular biological research.

Monoclonal antibodies are used in various forms by companies such as Vaccinex to treat an increasing number of conditions such as cancer. Vaccinex is also enrolling volunteers in a VX15/2503 trial for multiple sclerosis. Vaccinex believes the very same compound might help alleviate a host of other neurodegenerative diseases.

"The thought is that if we could give this drug early enough we can actually slow the progression of Huntington's, and that's really exciting," said Jody Corey-Bloom, M.D., Ph.D., the director of the Huntington's Disease Society of America Center of Excellence for Family Services and Research at the University of California, San Diego. She spoke to more than 50 people attending her annual HD research update presentation at the San Diego support group on October 26.

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Jody Corey-Bloom, M.D., Ph.D. (photo by Gene Veritas)

Aiming to halt progression, delay onset

Dr. Corey-Bloom, who's provided research updates to the support group since 2005, kicked off this year's presentation with a review of the historic news on October 19 that HD patients in England had received the first dosing of an Isis Pharmaceuticals drug, ISIS-HTT_{Rx}, aimed at stopping the disease at its genetic roots. ISIS-HTT_{Rx} is an antisense oligonucleotide (ASO), an artificial strand of DNA (click here to read more).

SIGNAL, initiated in June, is the "biggest" news on the HD clinical trial field scene since the development of ASOs for use in HD, Dr. Corey-Bloom said. SIGNAL marks the first ever use of a monoclonal antibody in an HD clinical trial, she noted.

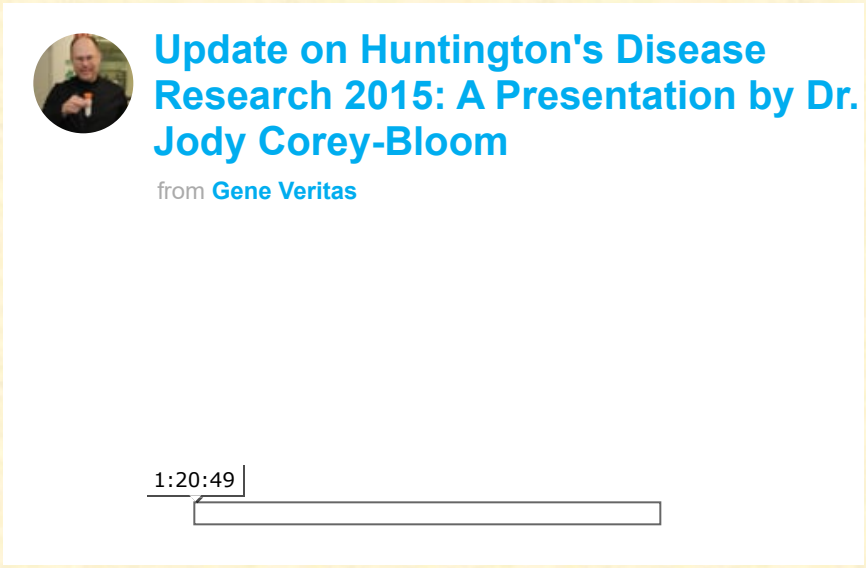
"So we're not just treating motor signs," she said of SIGNAL. "We're actually trying to slow the progression. And the reason is that this also belongs to a class of drugs that blocks inflammation in the brain in animal models. And so the hope is that we could either delay the onset or slow the progression."

VX15/2503 would help HD patients by reducing inflammation in the brain. Scientists primarily from the lab of world-renowned HD expert Michael Hayden, M.D., Ph.D., in collaboration with Vaccinex researchers, published a breakthrough study in April in the journal *Neurobiology of Disease* demonstrating how the use of a monoclonal antibody restored health in HD mice.

Vaccinex is one of a growing number of biotech and pharmaceutical companies that have delved into the search for HD treatments as research has greatly expanded.

As the scientist-written HD research site HDBuzz noted last February, other investigators are seeking ways to use other antibodies in the quest for treatments.

You can watch Dr. Corey-Bloom's research update, which includes details on numerous other hopeful projects, in the video below.



Update on Huntington's Disease Research 2015: A Presentation by Dr. Jody Corey-Bloom from Gene Veritas on Vimeo.

Including presymptomatic volunteers

HD clinical trials have rarely included presymptomatic people because of ethical concerns and the inability of science to measure a meaningful effect.

However, SIGNAL includes presymptomatic individuals for several reasons, in part because ways of detecting and interpreting the symptoms have become ever more effective, Dr. Corey-Bloom explained.

"You have to be gene-positive, but you don't even have to have a diagnosis, because they believe that the drug itself is not going to hurt you," she said, adding that the current stage of the SIGNAL trial (Phase II) is primarily to confirm its safety. (Later a Phase III would test the drug's efficacy.)

SIGNAL will evaluate changes in participants' brains using "very unique, very high-level, high-quality imaging," she said. "They're doing very special PET studies, and they're also doing very special MRI studies, DTI, diffusion tensor imaging, and so just a lot of very, very special techniques that are being orchestrated by the Massachusetts General Hospital and [researcher] Diana Rosas."

Measuring the effects

One attendee asked specifically how a presymptomatic individual would know whether onset had been delayed.

"I think the imaging data is going to be really compelling," Dr. Corey-Bloom said, noting that previous research has abundantly demonstrated changes in the brain a decade or more before onset, as well as precise measurements in those changes over time. "I think they're going to be able to tell that it isn't declining the way people who aren't being treated is declining.[...] If you're in the early stages and stay there, that would be pretty impressive, too."

Involving 36 volunteers at 13 sites across the country, SIGNAL will deliver VX15/2503 intravenously once per month over twelve months in one group and over 18 months in a second group. Dr. Corey-Bloom, whose Center of Excellence enrolled the first patient in SIGNA, said that each

infusion would last about an hour. (Click here for the official details of the trial, which will be administered by the Huntington Study Group [HSG]). The HSG just completed its 2015 meeting, held October 21-24 in Tampa, FL.

Further information about SIGNAL in the San Diego region is available at 858-246-1254.

Confidence, but....

I will call that number very soon to learn more about my eligibility and the risks involved.

I tested positive for HD in 1999, and my mother died of the disorder in 2006 at the age of 68 after a two-decade battle. I'm almost 56, a stage where my mother already had involuntary movements and suffered from cognitive loss.

After attending the HD support group and seeing symptomatic friends, I'm always worried about onset.

Dr. Corey-Bloom's recap of the good news about the long-awaited Isis clinical trial left me with a feeling of confidence that *someday* we will defeat HD – but perhaps not in time to stop my onset. The Isis trial does not include presymptomatic individuals, and, even if successful, it could take five, ten, perhaps even 20 years for the approach to have a significant impact on the disease.

Wishing for a 'normal' life

However, after hearing about SIGNAL for the first time, a flood of new emotions began to pour over me.

I immediately felt hope for my friend Sharon Shaffer, my HD sister, and other HD-affected individuals in the audience.

Sharon Shaffer and mother Fran Walker (photo by Gene Veritas)

I awoke at 3:45 the next morning full of energy. Unable to sleep again, I worked on processing the video of Dr. Corey-Bloom's talk.

"My 20th year attending support group – what a difference!" I wrote in my notes. "Treatments and trials, people asking questions about real scenarios, not just long-off hypotheses. PLUS: I learned of a trial that I can maybe take part in and see symptoms prevented. What if my career can be 'normal' and my life 'normal'?"

I've hardly ever had such positive thoughts, although I recognize that I am extremely lucky to have remained asymptomatic this long.

Risks vs. benefits

My wife, who has seen so many of her own plans dashed because of HD, was pleased to hear about SIGNAL.

She hopes every day for a treatment to save me, as well as others, from the devastation she witnessed in my mother.

As I gather more information in the coming weeks, and if I am eligible to participate in SIGNAL, I will weigh risks and benefits with her, my physicians, and members of the HD community.

My immediate concerns: could VX15/2503 cause harmful side effects or even trigger HD? Would participation somehow prevent me from taking part in other trials in the future?

'Unimaginable scenarios'

Regardless of my participation, I will follow this project with keen interest – as surely will the rest of the HD community.

As with the Isis compound, there is no guarantee VX15/2503 will work.

However, it is yet another shot on goal in the search for effective treatments. The more shots, the better the chance of success.

The HD community can now envision scenarios unimaginable 20 years ago. That's significant progress.

Posted by Gene Veritas at 12:13 AM

Labels: antibody , clinical trials , Huntington's disease , inflammation , Isis Pharmaceuticals , Jody Corey-Bloom , monoclonal antibody , mother , onset , presymptomatic , research , SIGNAL , symptoms , treatments , Vaccinex , VX15/2503

2 comments:

Unknown said...

Dear Ken,

SIGNAL is indeed exciting news! Gary and I continue to send our most positive thoughts and warmest wishes your way. We are sure that you will make the optimal decision as to whether you will take part in the clinical trial, and we remain steadfastly in your corner in your fight against HD! We are also convinced that life is full of miracles and, with the aid of scientific breakthroughs, you will be able to fend off this disease and be the ultimate winner!

8:00 AM, November 02, 2015

Unknown said...

Hi Gene Veritas! How are you?
My name is Bruna and I am Brazilian, I have 23 years old. I came to Toronto to study English. I live for years seeing the disease. In addition to the great-uncles more I live with my paternal grandmother, she has the disease and I have an aunt(I have 2 cousins in the group of risk, they didn't do the test yet) and my father was also diagnosed. So I live with this disease so closer. And as you know I can have the disease but I didn't do the test, even my sister. Despite being a sad disease, I could get more closer to my family because of it. And see how much my family is wonderful! As I will be a long time away and I saw the opportunity to read more about the disease here in North America and when I saw your blog and your history I felt really thrilled. I'm sure that you are doing your best and helping the world. I would like to say thank you for all the blog and everything that I saw you've been doing. Also,if I could help with something I would do with all my pleasure. I would love to know more about how the things here are different for disease from Brazil. My email is bruna_bernardino@hotmail.com | bruna.a.bernardino@gmail.com. Thank you. (sorry if I wrote some mistake in English haha learning)

8:29 PM, December 13, 2015

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