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CHDI's 13th conference promises some good news for the Huntington's disease community

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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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WEDNESDAY, FEBRUARY 21, 2018

CHDI's 13th conference promises some good news for the Huntington's disease community

With potential Huntington's disease treatments on the horizon, I am looking forward to the 13th Annual HD Therapeutics Conference with great anticipation.

Although a powerful reminder of my gene-carrier status, the opportunity to watch world-class academic and pharmaceutical researchers present their latest findings always leaves me with increased hope that I won't die from HD like my mother.

Sponsored by CHDI Foundation, Inc., the conference takes place at the Parker Palm Springs hotel in Palm Springs, CA, February 26-March 1. It will be my seventh, including an appearance as the keynote speaker in 2011.

The conference will assess progress towards HD treatments and point to future paths for research and clinical trials.

Gene Veritas (aka Kenneth P. Serbin) before the CHDI logo in 2012 (photo by Lev Blumenstein)

A report on the Ionis trial

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I'm especially eager to learn about the latest data from the highly successful Ionis Pharmaceuticals Phase 1/2a gene-silencing clinical trial, aimed at reducing the amount of harmful huntingtin protein in brain cells. The project received an initial infusion of about $10 million from CHDI, later repaid by Ionis.

With the end of Phase 1/2a last December, Ionis officials commented only briefly on the demonstrated safety and tolerability of its gene-silencing drug IONIS-HTT_{Rx}, reserving a more thorough update for scientific meetings.

In the final talk – perhaps saving the best for last – IONIS-HTT_{Rx} trial administrators Anne Smith, Ph.D. (Ionis) and Sarah Tabrizi, FRCP, Ph.D. (University College of London) will present "Development of IONIS-HTT_{Rx}: From first principles to the first successful HTT-lowering drug trial."

I'm hoping that Drs. Smith and Tabrizi will elaborate on the brief report from Ionis scientists in December that the drug did indeed safely and substantially lower the amount of mutant huntingtin protein, as measured in trial volunteers' cerebrospinal fluid. That was only a first step, but an important one.

As a result, the Ionis scientists stated, clinical trial partner Roche, now the license-holder for IONIS-HTT_{Rx}, could skip the usual Phase 2 of the clinical trial program, going directly to a full-blown Phase 3. Phases 2 and 3 measure a drug's efficacy. In consultation with the Food and Drug Administration (FDA) (and regulatory agencies in other countries), a drug company can shape the trial program as it sees fit.

I plan to interview Roche officials in Palm Springs, including Thomas Wiese, M.D., the Patient Partnership Director for the firm's HD program.

'The more the merrier'

"Wow, this is truly big news and very exciting news for the whole HD community since it tells us that the drug can do what it is designed to do!" HD specialist Jody Corey-Bloom, M.D., Ph.D., commented via e-mail in mid-December regarding the trial. "Now the hard part – can it make HD better?"

Also impressed, Martha A. Nance, M.D. pointed to the need for deeper clinical research: "Will people taking this treatment stabilize clinically, or improve? And what about the long-term safety? Will there be any unanticipated problems six months or five years later? These are the questions that will require additional larger studies to answer, before the drug is understood well enough to use as a treatment in the clinic."

"The IONIS results are ushering in a new and more hopeful era," remarked LaVonne Goodman, M.D., the founder of HDDrugWorks. "With the demonstrated safety (thus far) of the drug, and the urgency of our Huntington's family needs, it makes a lot of sense to be going next to a Phase 3 trial. Let's hope the regulators think so too."

Dr. Goodman added that the Cambridge, MA-based Wave Life Sciences is also currently enrolling patients in clinical trials of two gene-silencing drugs that, like Ionis's effort, use strands of artificial DNA known as antisense oligonucleotides (ASOs) to decrease the huntingtin protein.

She reflected on the fact that, the greater the number of clinical trials, the greater the chances of finding effective treatments for this still untreatable condition.

"Competition is good," Dr. Goodman said. "The more the merrier."

Wave's unique approach

Just as the Ionis trial has made history, so might the Wave program. Whereas IONIS-HTT$_{Rx}$ reduces both the mutant *and* normal huntingtin that all HD patients have, Wave's ASOs attack just the mutant by targeting two specific genetic variations found in 70 percent of the HD population.

"We're able to use the Wave technology to selectively lower the mutant huntingtin, while leaving the wild type, or healthy, huntingtin relatively alone," explained Wave HD researcher Serena Hung, M.D., in a February 7 webinar sponsored by the Huntington's Disease Society of America (HDSA) (click here to view). "This is a very unique approach, and this is probably the main difference between the Wave approach and other approaches."

So far, the trial is enrolling participants in Canada and Poland. During the webinar, Dr. Hung indicated that other countries could be included, but did not mention the U.S. (Recruitment information for clinical trials worldwide is available at https://clinicaltrials.gov/).

For years, researchers have studied and debated the benefits and drawbacks of these different approaches. The distinctive IONIS and Wave trials could provide valuable answers.

At the CHDI conference, Wave's Michael Panzara, M.D., MPH, will give a presentation on the firm's program immediately before the talk on the Ionis effort.

Landmarks and a lucky year

In his welcome letter to conference participants, CHDI Chief Scientific Officer Robert Pacifici, Ph.D., notes that 2017 marked HDSA's 50th anniversary.

"This year [2018] also marks the 25th anniversary of the identification of the huntingtin gene, a landmark accomplishment (for biology generally, not just HD) that of course could not have been achieved without the generous participation of HD families who volunteered their family history and donated their DNA."

Dr. Robert Pacifici (photo by Gene Veritas)

The participation of thousands of HD family members in genetic research and other projects has helped shape CHDI and the HD field's focus in the quest for treatments. That includes the "exciting new area" of DNA repair and handling, a theme of this year's conference.

In addition to the popular, ever-expanding conference poster session, this year's conference will include a resource fair, an innovation introduced at the 2017 conference in Malta. (Every fourth year, the conference is held in Europe.) The fair will display scientific tools and technologies useful in HD research.

"We are delighted to return to our 'home' here at the Parker Hotel in Palm Springs for lucky number 13!" Dr. Pacifici quips in his letter.

I officially became an advocate two decades ago this April by joining the board of HDSA-San Diego, where I served for twelve and a half years.

Blessed to have avoided symptoms so far, and observing the vast progress in HD research over these past two decades, I believe that 2018 could indeed be a lucky year for our community.

(Disclosure: I hold a symbolic amount of Ionis shares.)

Posted by Gene Veritas at 4:18 PM

Labels: CHDI Foundation , gene silencing , gene-carrier , HD Therapeutics Conference , Huntington's disease , Ionis Pharmaceuticals , IONIS-HTT-Rx , Parker Palm Springs , researchers , Roche , treatments , Wave Life Sciences

1 comment:

BJ V said...

Ken - thanks for the write up. I'll be in Palm Springs next week representing HDYO and the young people worldwide impacted by HD. I look forward to connecting sometime during the conference! -BJ Viau

5:32 PM, February 21, 2018

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