2-27-2018

Overflow audience at 13th annual Huntington's Disease Therapeutics Conference

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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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TUESDAY, FEBRUARY 27, 2018

Overflow audience at 13th annual Huntington's Disease Therapeutics Conference

With more than 350 Huntington's disease researchers and pharmaceutical executives in attendance, the 13th annual HD Therapeutics Conference got underway last night in anticipation of key presentations on progress towards treatments, including the successful Phase 1/2a Ionis Pharmaceuticals gene-silencing clinical trial completed late last year.

Sponsored by CHDI Foundation, Inc., the nonprofit virtual biotech focused on the search for HD treatments, the conference runs through March 1 at the Parker Palm Springs hotel (which is less than three hours' drive from my San Diego home). In the last presentation, Ionis clinical trial administrators will present the results of the Phase 1/2a study of the company's HD, drug IONIS-HTT_{Rx}.

"The first day we opened up the registration, we were almost full at the Parker immediately," Robert Pacifici, Ph.D., CHDI's chief scientific officer, said in his opening remarks in the main conference room, which holds 300 people. "We actually ended up allowing 365 participants to register. Not everybody can fit in this room." So, for the first time at the Parker, CHDI arranged for an overflow room, with a screen projecting the proceedings.

After cancellations, a total of some 350 are expected to attend, one of the largest audiences in the history of the conference.

Dr. Pacifici also presented a check for $38,000 to Louise Vetter, the CEO of the Huntington's Disease Society of America (HDSA).

As a research organization, CHDI provides no family services such as support groups and care centers. Demonstrating their commitment to HDSA's mission in these areas – essential for developing treatments – Dr. Pacifici and five other riders (mainly from CHDI) raised the funds in a recent 100-mile biking competition in the Southern California desert.

You can watch Dr. Pacifici's introduction in the video below.

International Huntington Association
Huntington's Disease Drug Works
Huntington's Disease Lighthouse
Hereditary Disease Foundation
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CHDI Conference Opens with Overflow Crowd

from **Gene Veritas**

14:07

CHDI Conference Opens with Overflow Crowd from Gene Veritas on Vimeo.

'Huntingtin lowering' and other main themes

Scientists and patient advocates eagerly await the March 1 presentation of the Ionis Phase 1/2a clinical trial, in which IONIS-HTT_{Rx} successfully lowered the amount of the mutant huntingtin protein in participants' cerebrospinal fluid.

The trial aimed not to study efficacy but safety and tolerability. The next phases of the trial remain a major hurdle: to test whether the drug can actually alleviate or reverse symptoms.

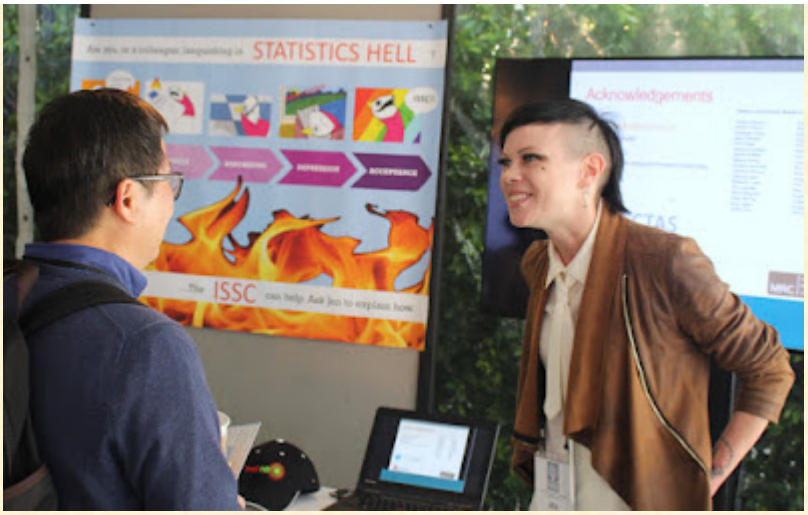
The Ionis contingent at the conference includes Frank Bennett, Ph.D., Ionis senior vice president of research and the franchise leader for the company's neurology programs, and Anne Smith, Ph.D., the Ionis director of clinical development and the individual responsible for the day-to-day management of the trial.

Dr. Smith will present the Ionis results along with Sarah Tabrizi, FRCP, Ph.D., of University College of London, the lead clinical trial site.

In addition to so-called "huntingtin lowering" strategies such as the Ionis drug, the main conference themes include potential therapies for fixing brain circuitry; the use of stem cells to better understand HD and develop treatments; the interplay of the huntingtin gene and DNA dynamics; and huntingtin protein structure and function.

The opening day also featured a resource fair, with research tools and databases available for HD research and developed by CHDI, partner organizations, and contract research organizations.

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



Jen Ware, Ph.D., CHDI's director for experimental design (right), explains a new CHDI research resource, the Independent Statistical Standing Committee, intended to provide independent, unbiased evaluation and expert advice regarding experimental design and statistics (photo by Gene Veritas).

Posted by Gene Veritas at 11:31 AM

Labels: CHDI Foundation , clinical trials , DNA , genetics , HDSA , huntingtin lowering , Huntington's disease , Ionis Pharmaceuticals , IONIS-HTT-Rx , Parker Palm Springs , pharmaceutical industry , Robert Pacifici , stem cells

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