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Top researcher: ‘Genuine optimism’ about treatments for Huntington's disease

Kenneth P. Serbin
University of San Diego

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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Top researcher: 'Genuine optimism' about treatments for Huntington's disease

With several potential treatments heading for clinical trials, the head researcher of the so-called "cure Huntington's initiative" feels "genuine optimism" about alleviating the devastating symptoms of this incurable, fatal brain disorder that also affects other areas of the body.

"People ask me all the time, 'Are you optimistic?'" said Dr. Robert Pacifici, the chief scientific officer of CHDI Management, Inc., the virtual biotech firm dedicated exclusively to ending HD. "I am. I really am. I'm genuinely optimistic."

CHDI Management is the research arm of the CHDI Foundation, Inc., the multi-million-dollar initiative backed by a group of anonymous donors.

Dr. Pacifici made his comments in an interview with me during CHDI's 7th Annual HD Therapeutics Conference at the Parker Palm Springs hotel in Palm Springs, CA, February 27-March 1.

Representing both academia and drug companies, the approximately 200 scientists and observers at the conference were abuzz with the promising developments presented by their colleagues in the formal part of the program.

When he expresses optimism, Dr. Pacifici told me, "the HD community doesn't let you get off easy. They say, 'Why?"

He outlined three major reasons for his optimism.

First, CHDI and the HD research community have "a large number of shots on goal" in terms of potential treatments.

"There are now eight things with the potential to reach the clinic in a two-year time horizon and a bunch more behind that," Dr. Pacifici said.

(Scientists do not predict the outcomeof trials nor the date at which an effective treatment will be found.)

Secondly, he stated that researchers will test each compound under study so as to obtain "an unambiguous result." This will permit the researchers to quickly evaluate the results and, if necessary, adjust the compounds.

Third, Dr. Pacifici pointed out that the potential treatments are "custom-crafted" for Huntington's disease.

Researchers know that potential drugs must to enter the brain, be taken for a long time, and not present serious negative side effects, he explained. By making the drugs HD-specific, the researchers can meet these challenges.

HD Links

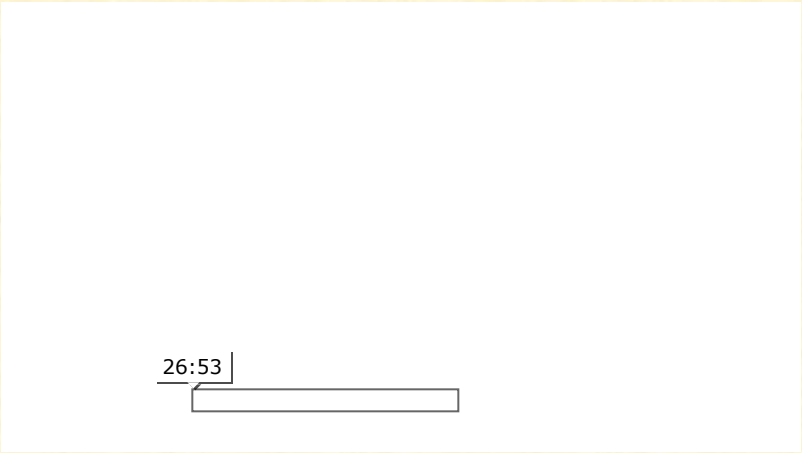
Huntington's Disease Society of America
International Huntington Association
Huntington's Disease Drug Works
Huntington's Disease Lighthouse
Hereditary Disease Foundation
Huntington's Disease Advocacy Center
Thomas Cellini Huntington's Foundation
HDSA Orange County (CA) Affiliate
HD Free with PGD!
Stanford HOPES
Earth Source CoQ10, Inc.

HD Blogs and Individuals

Chris Furbee: Huntingtons Dance
Angela F.: Surviving Huntington's?
Heather's Huntington's Disease Page

Future articles will explore the results of the conference and specific treatments in greater detail.

You can my watch the interview with Dr. Pacifici in the video below. Also visit www.hdbuzz.net.



Posted by Gene Veritas at 9:37 AM

Labels: biotech , brain , CHDI , clinical trial , cure , Huntington's , research , researcher , Robert Pacifici , scientist , treatments

2 comments:

Unknown said...
Great job Gene, this would be great to show at the support group meeting. Liked the way you got Dr. Pacifici to explain in common terms.

7:56 AM, March 02, 2012

Anonymous said...
Dear Sir .. I was very optimistic when I read this news .. But in recent times read that Alnylam has moved back from RNAi clinical trails .. Is there any other company persuing RNAi for Huntington's Disease? It would be great if you write something on this hopeful topic .. Thank You

12:52 AM, May 16, 2012

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