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‘Darkness replaced by hope and light’: taking stock of Huntington's disease 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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'Darkness replaced by hope and light': taking stock of Huntington's disease research

As the Huntington's disease community in 2015 enters a promising phase of clinical trials for remedies that might slow or halt the progression of the disease, one of the world's leading HD scientists recently took stock of the significant progress made over the past several decades.

"The horizons for therapy were very far away," Michael Hayden, M.D., Ph.D., the President of Global Research and Development and Chief Scientific Officer of Israel-based Teva Pharmaceuticals, said during a February 10, 2015, presentation about his company's latest efforts to defeat HD.

Dr. Hayden recalled how, 40 years ago, HD was virtually unknown in his native South Africa and many other countries. HD-afflicted people faced stigma and were left to cope with their disease "in isolation and despair," Dr. Hayden remembered of his first contact with the disease as a young medical student.

"It was just a dream then that there would be pharmaceutical companies interested in Huntington disease," he said. (In some countries HD is referred to as "Huntington," not "Huntington's.")

However, as Teva and other companies work towards HD treatments, the outlook has changed dramatically.

"We are now in a place of really tremendous hope," Dr. Hayden declared enthusiastically. "The darkness is replaced by hope and light. At the end of the tunnel we are seeing this light now, not only with this [Teva's] drug but other trials you are hearing about."

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

Dr. Michael Hayden (photo by Gene Veritas)

Reexamining a promising drug candidate

Dr. Hayden offered these remarks during a Huntington's Disease Society of America (HDSA) research webinar titled "Pride-HD: a dose finding, safety and efficacy study of pridopidine in HD patients." (Click here to watch the webinar.)

Pridopidine was first tested in two clinical trials – in Europe and North America – by the Danish pharmaceutical company Neurosearch. The company, although it observed some interesting effects, did not achieve sufficiently positive results to bring the drug to market.

That's because the chosen "endpoint" in the study, the way of measuring the drug's effects, didn't show a result significant enough for obtaining regulatory approval, Dr. Hayden explained. Neurosearch chose only a subset of motor symptoms as the study endpoint.

But some researchers still believed pridopidine had potential as an HD drug. Dr. Hayden and Teva studied the overall impact of pridopidine and, after obtaining the license for the drug from Neurosearch, have decided to run an additional clinical trial, called Pride-HD (Pridopidine Dose Escalation in HD). Teva is using a different endpoint, the so-called total motor score, a measurement of *all* the motor symptoms.

Dr. Hayden observed that patients in the earlier pridopidine studies actually showed improvement in motor symptoms caused by HD such as chorea, or involuntary movements. Pridopidine also improved eye movement substantially, he said. It also stabilized levels of dopamine, a neurotransmitter and hormone involved in movement control, mood, and motivation.

Patients' depression, another telltale HD symptom, also did not worsen, Dr. Hayden noted.

Aiming for broader impact

"There may be some broader affect on other features of Huntington disease," he added. Additional studies of pridopidine in animals have indicated that it brings about changes in the brain, might be "neuroprotective," and might help with improving thinking and feeling, he explained.

Therefore, Teva will add other key endpoints to the Pride-HD study: cognition, mood, and quality of life. The study also will assess the effect of dosages of pridopidine higher than those given patients in the earlier trials.

Pridopidine "could theoretically have some effect to change the course of the illness," Dr. Hayden observed. "And wouldn't that be exciting?"

Pride-HD enrolled its first patient in April 2014 and will continue throughout 2015. Teva aims to enroll 400 patients at 54 sites in North America, Europe, and Australia. If the results are favorable, Teva could seek regulatory approval for the drug in late 2016, Dr. Hayden said.

As HD specialist Dr. LaVonne Goodman noted in her February 18 commentary on the potential of pridopidine, "recruitment is not going well for Pride-HD.[...] The bottom line is that finding new drugs for HD takes a lot of work, good trials and a long-term commitment from HD families and investigators. If we don't join this or other trials, we will never have new drugs for HD: not for ourselves or the next generation."

To learn more about Pride-HD and how to participate, refer to the above-mentioned link to the webinar or call HDSA at 800-345-4372.

Teva is also conducting a clinical trial of the drug laquinomod for use in HD, Dr. Hayden noted. Laquinomod is thought to reduce the inflammation of the brain in neurological disorders. Click here to learn more about the trial.

Another historic moment

As a carrier of the HD gene mutation, I listened to Dr. Hayden's comments on the long-term progress of HD research with great hope.

Scientists have observed that managing HD effectively likely will require a cocktail of drugs. Pridopidine is yet another potential element in the mix. (I have reported on many of the elements in this blog over the past ten years. Click here and here to see recent examples.)

Having tracked the HD movement for nearly 20 years, I also appreciated Dr. Hayden's important reminder that the quest for effective treatments is a lengthy process. Science and clinical trials require time and investments of money and intellect.

I wrote this article in Palm Springs, CA, just before the start of yet another historic mark in the HD movement: the 10th Annual HD Therapeutics Conference, sponsored by the CHDI Foundation, Inc., at the Parker Palm Springs hotel February 23-26. In the past I have referred to this event as the "Super Bowl" of HD research.

In Palm Springs, I will listen to other scientists take stock of the search for HD treatments.

I also expect to witness yet further examples of researchers replacing the darkness of Huntington's disease with hope and light.

For yet more perspective, watch my interview with Dr. Hayden at the 2011 HD Therapeutics Conference in the video below.

Gene Veritas interviews Huntington's disease expert Michael Hayden

from **Gene Veritas**

38:25

Gene Veritas interviews Huntington's disease expert Michael Hayden from Gene Veritas on Vimeo.

Posted by Gene Veritas at 9:26 AM

Labels: CHDI , clinical trials , cocktail , dopamine , HD Therapeutics Conference , hope , Huntington's disease , Michael Hayden , pridopidine , research , scientists , symptoms , Teva Pharmaceuticals , total motor score , treatments

1 comment:

Anonymous said...

Hi, "Gene Veritas".

I´m from Brazil and, well, we (I and you) have the same problem: risk of HD.

(Sorry by my so so english).

Sometimes, I read your blog. It´s very rich and hopeful. Great Job!

I hope the HD will be stopped soon (maybe until 10 years??).

It's good to know that great scientists of world are working hard to "solve" this problem ("HD").

Which God enlighten every one.

Bye!

8:20 PM, March 24, 2015

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