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Time for optimism: a cure for Huntington's could be near

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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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GENE VERITAS

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SUNDAY, APRIL 06, 2008

Time for optimism: a cure for Huntington's could be near

On October 26, 2007 a California pharmaceutical company announced a multi-million-dollar project to develop a drug that would eliminate the root cause of Huntington's disease.

If it works, the project would not only cure Huntington's but could revolutionize treatments for other diseases and usher in a new era of medical advances that just a few years ago seemed like science fiction.

<u>Isis Pharmaceuticals</u>, <u>Inc.</u>, located in the San Diego suburb of Carlsbad, revealed that it is working on an antisense drug for HD. This class of drugs is designed to block the action of genes that cause disease. In the case of HD Isis aims to stop the huntingtin gene from making proteins that disrupt brain cells and cause the harmful symptoms of HD.

An impressive development

In my opinion this is the most impressive and promising initiative ever developed for finding a treatment or cure for HD. Isis is aiming for results *now* and in humans, not just in a test tube or in a mouse.

The technology is based on the discovery of RNA interference, or RNAi. RNAi is a natural process in which genes are switched off. Its discovery was considered the top scientific breakthrough of 2002, and scientists have been studying it intensively ever since. So are some drug companies.

Isis has already gotten approval from the U.S. Food and Drug Administration (FDA) for Vitravene, which it calls the "world's first antisense drug" to go to market. Vitravene is used to treat an eye disease associated with AIDS.

And Isis has used an antisense drug to reduce the effects of Lou Gehrig's disease in test rodents. Isis delivered the drug, ISIS 333611, directly into the rodents' spinal fluid via an implantable pump.

A pump in the brain

I have long imagined that this could be the future for me and many hundreds of thousands of other people around the world who are genepositive for HD: we would have a small pump on or in our heads delivering a life-saving drug to our brains. HD would be totally controlled.

Such pumps now exist because of nano-technology and have actually been used safely in thousands of people. Doctors surgically insert the device into the brain. They inject medication into the brain during a routine visit to the doctor's office.

It is especially encouraging that Isis has already shown that it could reduce the action of huntingtin in the brain and peripheral tissues of normal mice **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.

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

using an antisense compound.

\$9.9 million in funding

The Isis research is backed by the <u>Cure Huntington's Disease Initiative</u>, Inc. (CHDI), a recently founded drug discovery firm targeted exclusively at HD and funded by a private, anonymous donor. Based in Los Angeles, CHDI will provide Isis up to \$9.9 million for the project. This is one of the largest amounts - if not the largest - ever spent on an HD research project.

The project will first focus on testing an antisense drug in transgenic HD mice - genetically engineered animals that have the same genetic defect as human HD patients. If successful, Isis will then test the drug in monkeys. CHDI could then approach the FDA for approval for human testing as early as the third year of the project.

"This is very good news because a potent RNA drug will stop Huntington's at its source, and we could let ourselves say the 'cure' word," Dr. LaVonne Goodman, the founder of Huntington's Disease Drug Works and an expert on HD research, wrote shortly after the Isis announcement. "I confess that just a year ago I didn't believe I'd see this much progress on RNA therapy in my lifetime, and I'm glad I'm wrong."

Reversing HD's devastating effects

If successful, the Isis antisense drug could very well do more than prevent HD: it might also partially reverse the disease. Studies of RNAi treatment in mice have shown that the animals recovered normal motor function because of the ability of brain cells to regain health and take over the job of cells destroyed by HD.

This possibility brings me a mixture of joy, frustration, sadness, and guilt. I am excited to know that if my own symptoms start soon, the potential Isis drug may be able to stop them and keep my brain completely healthy. And I am happy that acquaintances with HD might return to a normal life and save their families from witnessing their horrible decline. But I also wish that such promise had existed two years ago February, when my own mother died of HD.

I learned about my mother's HD in 1995, and I tested positive in 1999. Living at risk for HD – a 100-percent genetic disease that affects all genepositive individuals - has impacted every aspect of my and my family's lives. It has been extremely frustrating to see scores of theoretical advances over the years but no real hope of an effective treatment.

Time for optimism

Now, for perhaps the first time, I am beginning to feel optimistic about my future and that of the HD community. As I wrote recently (*click here to* <u>read more</u>), in December the state of California moved a step closer to considering a multi-million-dollar project to create a program to seek a treatment for HD using stem cells. Other scientific breakthroughs continue to occur.

I have felt especially moved to express optimism because in the last few days I have felt deeply sad at reading about young people with HD at the new website WeAreHD.org. They are struggling with symptoms and worrying about the fate of their potentially at-risk children.

The HD community needs to support the Isis-CHDI partnership in any way it can. I will visit Isis very soon to learn more about its HD project, so please stay tuned for a new entry on this extremely important topic.

Posted by Gene Veritas at 4:23 PM











8 comments:

Anonymous said...

Upon researching Huntington's disease online for my research assignment, I managed to stumble upon this page.

It's saddening how Huntington's is like a ticking time bomb, but at the same time, I am marvelled at the advances in modern medicine which have improved the lives of everybody, and is continuing to do so.

I believe a cure for Huntington's is inevitable; perhaps more propitious than well-known AIDS.

3:27 AM, September 08, 2008

Management States Anonymous said...

You have a beautiful blog here. It's wonderful to hear about treatments that may become available in the near future. I am at risk for Huntington's (I refuse to be tested until there is a treatment to slow or stop the decline, I do however take all the vitamin supplements recommended for slowing the disease). It breaks my heart to hear everyone's stories about Huntington's but in reading them I find a small comfort that others have the exact fears that I have.

My grandmother had a total of 7 children, 2 died before they were 30 so we don't know if they were positive and the other 5 all have Huntington's or have died from Huntington's. My grandfather was the carrier so my grandmother has had to watch 6 of her children die and my father is now in later stages of the disease.

When Huntington's is aired on a television show it angers me how the main thing they talk about is the physical symptoms. I could handle the knowledge of my body being beyond my control if I did not also worry about my mind going, everytime I act unreasonable I'm sure it's signs of Huntington's, I wish more people understood that fear.

Thank you for your work on this wonderful and soulful page.

8:56 PM, March 06, 2009



Walter Market Market

My husband started to show the symptoms. He doesn't realise that he has it.

His mother had. I have been crying since then when he is not around, because I can't imagine a life without him.

I love him so much, he is my light, my soul. He is so young. When I found out about research and the cure I can sleep at nights.

Thank you for giving me hope.

6:04 PM, June 17, 2009

Anonymous said...

As a possible carrier of the gene and a father of three beautiful children, I was ecstatic as I read this article. My brothers and I have all avoided testing to this point, but as time progresses that will of course have to change. we lost our father to HD two years ago. The daily worries of any little possible sign to confirm that ourself and possibly our children will have to face our end in this way is excruciating. I pray daily for a cure, and though we do no not have alot of money or anything, I will gladly do what I can to assist in stopping this disease from destroying the lives of myself, my brothers and our families-not to mention the aunts, uncles, cousins and everyone else affected by this disease. Please keep up the great work.

10:32 AM, March 13, 2010

& Katie said...

I am very glad to hear that there might be hope after all. I am also at risk for huntington's. My grandmother died from it before i was born. My father has been in a nursing home for 6 years now with it, my aunt died last june, and i am very very sad to say that my sister now is showing signs, shes 25 years old, and a mother of 2. and to know that there could be a way to save them..... is the greatest news iv ever heard. And im very thankful for sites like this so that i dont have to feel so alone..... 6:31 PM, April 11, 2010

Grant said...

This is some promising news, my aunty died a few days ago due to HD, My other aunty and uncle suffer with it, along with my mother. Me and my 2 sisters are all scared at the prospect of Having HD, And upon reading this blog you have given us a sense of hope, it may be to late for my aunt, but its not for the rest of us...Thank You

4:21 AM, February 20, 2011

Anonymous said...

I am so glad to hear of this news. My Grandmother was a carrier and I am starting to think my father at 43 is getting it. His leg bounces around while watching TV and his personality has changed a lot. He can no longer focus on one thing for very long and smokes a lot of cannabis. I was wondering if his dependance on cannabis could worsen the onset? I am young (20) and don't want to take the test but am worried if I was a carrier that I would pass it onto my children. Is anyone in a similar predicament? Im not sure if they could test sperm and inseminate without my knowledge of positive or negative HD sperm?

4:20 AM, April 23, 2011

Ali briceño said...