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News flash: Isis and Roche hope to start Huntington's gene-silencing trials in first half of 2015

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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The long-anticipated clinical trial of a drug that could potentially stop Huntington's disease at its genetic roots and perhaps someday even prevent the disorder in presymptomatic HD gene carriers like me could start by the middle of 2015.

If successful, the trial could result in a drug in five or six years.

Officials at Carlsbad, CA-based Isis Pharmaceuticals, Inc., in an interview with me on August 22, said that the Phase I trial for their drug, ISIS-HTT_{Rx}, likely will start by the second quarter of 2015, as long as the company receives regulatory approvals and fulfills other standard requirements for trials.

ISIS-HTT_{Rx} is an antisense oligonucleotide (ASO), a synthetic strand of DNA that silences, or turns off, the messenger RNA that makes proteins as coded by the DNA. If ISIS-HTT_{Rx} works as intended, it would reduce the production of the huntingtin protein in brain cells, reduce damage to the brain, and reduce or even eliminate HD symptoms.

ISIS-HTT_{Rx} is the company's internal name for the drug, which will later receive a generic scientific name and, if it reaches the market, a commercial name. HTT is scientific shorthand for the huntingtin gene, messenger RNA, and protein. Rx is shorthand for a medical prescription.

Isis is also conducting standard toxicological studies of the drug in non-human primates to assure that it will not cause harm to humans. A Phase I trial tests for safety and tolerability. Researchers can make observations about the drug's efficacy but must then conduct Phase II and Phase III trials, which involve more people, to demonstrate whether the drug really works.

Isis is planning the trial with the Swiss pharmaceutical giant Roche, vastly experienced in clinical trials and staffed with specialists in neurological disorders. Last year the two entered a partnership that included a $30 million infusion of funds into the Isis preparations for the clinical trial.

The trial will involve 36 early-stage Huntington's patients at four to six sites in Canada and Europe. If Phase II occurs, the companies would extend the study to the U.S.

Only recently did Isis, a world leader in ASO science and technology, settle on ISIS-HTT_{Rx}.

You can watch my brief report from Isis headquarters in the video below. Soon I will provide a detailed report on the ISIS-HTT_{Rx} clinical trial project.

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

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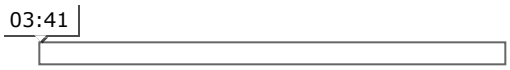
from **Gene Veritas**



News flash: Isis and Roche hope to start Huntington's disease gene-silencing trial in first half of 2015 from Gene Veritas on Vimeo.

Ramping up

I have tracked the Isis project since 2008 and, with the rest of the HD community, anxiously awaited the start of the ASO trial.

I became excited when I recently saw ISIS-HTT_{Rx} listed on the Isis website. It reminded me of the need to get an update on the project. This last visit to the company was my fifth.

At my first visit in 2008, I had learned that Isis hoped to start a Phase I trial in 2010. However, each time I obtained an update on the project, I learned that the researchers had postponed the start of the trial to account for new scientific discoveries, advances in HD research, improved ASO technology developed by Isis itself, and the desire to engineer the safest and most effective drug possible.

The postponements always disappointed me, but I also understood that scientific research and drug discovery are slow and painstaking processes.

However, during the August 22 meeting, it became abundantly clear that Isis and Roche are ramping up for the clinical trial. They are making necessary final arrangements such as the selection of sites, to be announced in early 2015. Significantly, with the selection of ISIS-HTT_{Rx} – the culmination of nearly a decade-long search for an efficacious drug in which the company tested some 2,000 ASOs – the engineering is complete.

Optimism and realism

Later that day, I pondered the likelihood of the Isis-Roche trial and how much of a change that meant for me, and for those in my situation.

After so many years of research and millions of dollars in investments, a clinical trial was becoming a reality.

Reviewing the Isis visit with my wife Regina during a late-afternoon walk, I mentioned how a future, improved version of ISIS-HTT_{Rx} might prevent HD symptoms.

At 54, I am now well past my mother's age of HD onset. Each day without HD is a gift. I felt simultaneously hopeful and concerned, optimistic and realistic, as Regina and I calculated when ISIS-HTT_{Rx} might reach the

market: Phase I would likely end in 2017, and Phases II and III would likely take the project beyond 2020. A second generation of drugs for asymptomatic gene carriers would come even later.

I recalled that a clinical trial is an experiment with an unpredictable outcome.

More than ever I need to focus on maintaining my health in order to postpone the inevitable HD onset as long as possible.

In the meantime, I will cheer on the Isis-Roche team as it brings the hope of an HD-stopping drug.

See below links to previous reports on Isis.

"A key new ally in the search for Huntington's disease treatments"

"Quickening the pace towards a Huntington's disease gene-silencing trial: pharma Giant Roche, Isis enter partnership"

"Designing the best drug possible to defeat Huntington's disease"

"Building a 'laser-guided' missile to defeat Huntington's disease"

"Observing the cure in progress"

Posted by Gene Veritas at 7:35 PM

Labels: antisense oligonucleotide , clinical trial , cure , disease , DNA , gene silencing , HD gene carrier , huntingtin , Huntington's disease , Isis Pharmaceuticals , ISIS-HTT-Rx , protein , RNA , Roche , treatments

5 comments:

Unknown said...
Hi Ken,

This is indeed wonderful news! And we applaud your persistent and energetic efforts at urging the local pharmaceutical companies to speed up their experiments with the promising treatment!

9:49 PM, August 23, 2014

Anonymous said...
This great news. I am so glad you've shared this update with us.
Lou

8:21 PM, August 24, 2014

Anonymous said...
"If successful, the trial could result in a drug in five or six years."
Does it mean that after succesful Phase 3, it is required to spen 6 years to deliver final product to the market?

5:04 AM, August 26, 2014

Paul van de Groep said...
This news give us hope for the furure! Thanks for sharing the news; we will follow you on Twitter @PaulvandeGroep

1:09 PM, August 26, 2014

Paul van de Groep said...

My Daughter-in-law (30 years old)has been tested positive last year. She has two little daughters. Her father is affected too.

11:08 AM, August 27, 2014

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