

University of San Diego

Digital USD

At Risk for Huntington's Disease

Department of History

2-25-2016

Chief Huntington's disease drug hunter: 'every confidence first treatments' in the works

Kenneth P. Serbin
University of San Diego

Follow this and additional works at: <https://digital.sandiego.edu/huntingtons>



Part of the [Nervous System Diseases Commons](#)

Digital USD Citation

Serbin, Kenneth P., "Chief Huntington's disease drug hunter: 'every confidence first treatments' in the works" (2016). *At Risk for Huntington's Disease*. 223.
<https://digital.sandiego.edu/huntingtons/223>

This Blog Post is brought to you for free and open access by the Department of History at Digital USD. It has been accepted for inclusion in At Risk for Huntington's Disease by an authorized administrator of Digital USD. For more information, please contact digital@sandiego.edu.

[More](#)[Create Blog](#) [Sign In](#)


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.

Blog Archive

- ▶ 2021 (12)
- ▶ 2020 (16)
- ▶ 2019 (19)
- ▶ 2018 (16)
- ▶ 2017 (14)
- ▼ 2016 (13)
 - ▶ November (2)
 - ▶ October (1)
 - ▶ September (1)
 - ▶ May (2)
 - ▶ April (2)
 - ▶ March (1)
 - ▼ February (2)
 - [Chief Huntington's disease drug hunter: 'every con...](#)
 - [The little things that are really big: caregiving ...](#)
- ▶ January (2)
- ▶ 2015 (24)
- ▶ 2014 (24)
- ▶ 2013 (30)
- ▶ 2012 (26)
- ▶ 2011 (33)
- ▶ 2010 (26)
- ▶ 2009 (21)
- ▶ 2008 (7)
- ▶ 2007 (7)
- ▶ 2006 (4)
- ▶ 2005 (17)

About Me

 [GENE VERITAS](#)

[View my complete profile](#)

HD Links

[Huntington's Disease Society of America](#)
[International Huntington Association](#)

THURSDAY, FEBRUARY 25, 2016

Chief Huntington's disease drug hunter: 'every confidence first treatments' in the works

Surveying the vast progress in Huntington's disease research and a "blitz" of clinical trials now in progress, a key scientific leader in the efforts has predicted that they will produce treatments for the incurable neurological disorder.

"I have every confidence that this batch of clinical candidates that are now being tested are going to yield the first treatments for Huntington's," said Robert Pacifici, Ph.D., the chief scientific officer for [CHDI](#), the multi-million-dollar non-profit virtual HD biotech.

Dr. Pacifici's remarks came in an interview on February 24 in Palm Springs, CA, at the organization's 11th Annual HD Therapeutics Conference, sponsored by CHDI Foundation, Inc., the backer of the initiative.

Nobody can foretell the result of a clinical trial, and Dr. Pacifici did not specify a timeline for an effective treatment reaching the market. However, he offered examples of the immense progress towards developing treatments.

"It's really frustrating for people, I know, to say, "That's good news, that's good news, but where's the treatment, where are the drugs?" Dr. Pacifici said. "The thing I would point to is that so many of the things we said were going to happen actually have happened. And so many of the things that have happened have actually yielded the outcome that we wanted."

Dr. Pacifici cited three recent key advances: the ongoing research in biomarkers (signals) to measure the efficacy of potential drugs' in reducing the harmful presence of abnormal huntingtin protein in brain cells; major progress in identifying modifier genes that delay or hasten disease onset; and the start of clinical trials.

The trials post the most "difficult" challenge in the process, he said.

"We want to make sure that we do things in a way that obviously is very careful," he said, explaining the primacy of drug safety. "The last thing we want to do is harm anybody."

Noting that the search for drugs has no guarantees, Dr. Pacifici nevertheless concluded that these are "exciting times" for the community of HD families, researchers, and supporters of the cause.

You can watch my interview with Dr. Pacifici in the video below.

[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](#)



'Every confidence first Huntington's disease treatments' in the works

from [Gene Veritas](#)

27:48

['Every confidence first Huntington's disease treatments' in the works](#) from [Gene Veritas](#) on [Vimeo](#).

First Ionis patients safe

Until recent years, such good news seemed like a remote possibility for HD Therapeutics Conference participants and, indeed, for the entire HD community.

Because of the growing number of HD clinical trials and therefore greater hope for effective treatments, Dr. Pacifici and fellow CHDI conference organizers launched a new feature at this year's conference, the "Clinical Trials Update Blitz." Representatives from four different trials presented their latest news in 15-minute presentations.

The most anticipated update focused on the [historic trial by Ionis Pharmaceuticals, Inc.](#), to attack the root cause of HD via gene-silencing.

Trial principal investigator [Sarah Tabrizi, M.D., Ph.D.](#), of University College London reported that all patients in the very first cohort in the Phase 1b/2a trial – aimed at testing primarily safety and tolerability – completed the trial without incident.



Dr. Sarah Tabrizi updating the historic Ionis gene-silencing trial (photo by Gene Veritas)

The first group of participants received the first dosing of the drug, IONIS-RTT_{RX}, in October 2015. They received three additional doses at 28-day intervals and were monitored by trial administrators.

“We completed Cohort A, in London and Vancouver, four subjects, and the DSMB [independent data safety monitoring review board] met and allowed us to move to cohort B,” Dr. Tabrizi said. (A DSMB, a standard in all clinical trials, halts a study if patient safety is threatened.)

Administrators of the Ionis trial are currently recruiting volunteers for Cohort B, to be followed by Cohorts C and D, as outlined in the plans for the experiment. In all, 36 patients will take part in Phase 1b/2a. If all cohorts are successful, Ionis will seek approval for a full-blown, larger Phase 2 trial to test drug efficacy.

For further background on the trial, watch Dr. Tabrizi's update in the video below.



First Patients Safe in Ionis Trial for Huntington's Disease Treatment

from [Gene Veritas](#)

17:47 |



[First Patients Safe in Ionis Trial for Huntington's Disease Treatment](#) from [Gene Veritas](#) on [Vimeo](#)

More than a disease

Drawing a record 325 participants from academia, the pharmaceutical business, and the medical field, the conference highlighted cutting-edge HD research, including the structure and function of the huntingtin protein; the huntingtin gene and the human genome; potential gene-silencing treatments; restoration of cell health; and ways to measure clinical trial outcomes.

Although highly dedicated to HD research, many of the non-physician scientists have little if any contact with HD families. This has prompted CHDI to open each conference with a keynote by a representative of the HD community to drive home the human reality of the disease and the urgent need for treatments. (I [keynoted](#) the 2011 meeting.)

This year Astri Arnesen and Svein Olaf Olsen, a married couple who have led the HD cause in their native Norway and in the [European Huntington Association](#), delivered a powerful keynote about HD and marital commitment, denial, genetic testing, and raising a family. They titled their presentation “HD – more than a disease!”

Astri and Svein Olaf received a standing ovation.

I will explore their story and provide an overview of the key scientific findings in a second report on the conference.



Svein Olaf Olsen (left) and Astri Arnesen (photo by Gene Veritas)

An upbeat mood

As in past years, the CHDI meeting moved me profoundly.

I identified with the many difficult feelings and experiences recounted by Astri and Svein Olaf.

Once again, the scientists' presentations reminded me of HD's devastation of the brain – and of my vulnerability as a carrier of the HD mutation.







But this was a very upbeat conference. I had never spoken before to Dr. Tabrizi, but we hugged as if we were old friends after I congratulated her on the initial clinical trial report. The HD community has waited so long for such news!

Later, after I worked late into the evening on this article and missed the buffet dinner, Jerry Turner, the CHDI staffer in charge of conference logistics, arranged for a care plate of sumptuous leftovers from the kitchen of the Parker hotel, the gracious host of the conference.

I toasted to the success of the Ionis clinical trial and to CHDI's commitment to the project with Doug Macdonald, Ph.D., CHDI's director of drug discovery and development and its point man on gene-silencing.

As I told another scientist, I look forward to the day when we can all toast the discovery of an effective treatment.

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

Posted by [Gene Veritas](#) at 11:36 AM      

Labels: [biomarkers](#) , [brain](#) , [CHDI](#) , [clinical trials](#) , [gene-silencing](#) , [huntingtin](#) , [Huntington's disease](#) , [Ionis Pharmaceuticals](#) , [modifier gene](#) , [neurological](#) , [onset](#) , [Robert Pacifici](#) , [Sarah Tabrizi](#) , [treatments](#)

1 comment:

 **Anonymous said...**

Gene, thank you so much for your updates. As an HD mutation carrier, I always look forward to these. I admire your dedication to get your article out asap, so much that you would miss your conference dinner and stay up late writing! There is certainly a lot of hope and a lot of good news to look forward to. Justine.

3:28 PM, February 25, 2016

[Post a Comment](#)

[Newer Post](#)

[Home](#)

[Older Post](#)

Subscribe to: [Post Comments \(Atom\)](#)
