

University of San Diego

Digital USD

At Risk for Huntington's Disease

Department of History

3-5-2019

'We can now fear Huntington's disease less': reflections on the 14th Therapeutics Conference

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., "We can now fear Huntington's disease less': reflections on the 14th Therapeutics Conference" (2019). *At Risk for Huntington's Disease*. 267.
<https://digital.sandiego.edu/huntingtons/267>

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)

- ▶ November (2)

- ▶ October (2)

- ▶ August (2)

- ▶ July (1)

- ▶ June (2)

- ▶ May (2)

- ▶ April (1)

- ▼ March (3)

[Roche: less frequent dosing for Phase 3 Huntington...](#)

[Roche ramps up Huntington's disease clinical trial...](#)

['We can now fear Huntington's disease less': refle...](#)

- ▶ February (2)

- ▶ January (2)

- ▶ 2018 (16)

- ▶ 2017 (14)

- ▶ 2016 (13)

- ▶ 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

TUESDAY, MARCH 05, 2019

'We can now fear Huntington's disease less': reflections on the 14th Therapeutics Conference

As in past years, covering [CHDI's Foundation's](#) recent Annual Huntington's Disease Therapeutics Conference in Palm Springs, CA, produced a [whirlwind of emotions](#) about the devastating disorder that took my mother's life and looms over the lives of tens of thousands of HD patients and presymptomatic gene carriers like me.

Last year, I left the conference during the Thursday night farewell dinner to drive 120 miles to my home in San Diego, arriving after 1 a.m. Wired from strong black tea and that evening's news that the initial Ionis-Roche clinical trial had reduced the amount of the mutant huntingtin protein in trial participants' cerebrospinal fluid, I worked until 5 a.m. on an [article](#) about the "best news for the Huntington's disease community since the discovery of the gene" in 1993.

This year I planned for a calmer post-conference moment by spending the last night in Palm Springs. That allowed me to enjoy and socialize at the dinner, get a good night's rest after the long, adrenalin-filled four-day meeting, and drive back to San Diego leisurely the next day. I believe that such self-care is important in avoiding disease onset.

The smoother transition back home has helped me reflect on the progress towards HD treatments and solidarity among affected families, advocates, scientists, and drug companies.

'Rod Man' and his family's fight

This was my eighth Therapeutics Conference since 2010.

This year's event, the 14th annual conference, opened on February 25 with the unusual and deeply moving keynote address by comedian [Rod "Rod Man" Thompson](#), the winner of season 8 (2014) of NBC's *Last Comic Standing* reality TV talent competition. Rod was the first African-American CHDI keynoter.

Raw and humorous, Rod's presentation contrasted sharply with the serious, more formal speeches of most previous keynoters, including [mine in 2011](#).

Rod described the terrible physical, cognitive, and psychiatric decline that HD has wrought in his 66-year-old mother Shirley, who lives in the small Georgia town of Villa Rica.

"I see depression and sadness about stuff that can be a misunderstanding to most people, and they'll let it go, but she still holds on to it and harbors it," Rod said.

Because of Shirley's involuntary movements, the family also keeps kitchen utensils away from her to prevent her from injuring herself or others.

[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](#)

“Now it’s better for her to eat with her hands, because she’s a little shaky,” Rod explained. “Her body’s not the same.”

I cried as I remembered my own mother’s depression and inability to care for herself. She died at age 68 after a two-decade struggle with HD.

Untested, Rod and his two daughters – a college graduate and a high school student – are also at risk.

Rod received a standing ovation.

“What a presentation,” CHDI Chief Scientific Officer Robert Pacifici, Ph.D., observed in his [closing remarks](#) on February 28. “It was heartfelt. It was pretty amazing to see, in his own quirky way, how he touched on so many of the things that are so important to families, how important our work is, how difficult it is for the caregivers, how much they’re counting on us to deliver something, how complicated the science is for people who are trying to struggle through with what it means in their lives, the information that needs to get to places, the difficulty of visiting the clinics, and the challenges of participating in some of the clinical trials.”

A future article will feature Rod’s speech.



Huntington's Disease Society of America CEO Louise Vetter with 2019 CHDI keynote speaker Rod "Rod Man" Thompson (photo by Gene Veritas)

Collaboration toward a common goal

I felt especially in sync this year with the scientists and fellow advocates. In our ultra-competitive society, facing HD has helped teach me the value of collaboration. At the conference, we all focused intensely on the common goal of developing treatments.

The HD community is known for the close cooperation between scientists and affected families. As one neurologist wrote me last year, when the scientific and medical leaders of the cause “get together, they by and large have tended to check their egos at the door and just try to do what needs to be done.”

At the conference, I paid special attention to the February 28 talk by Marcy MacDonald, Ph.D., a [pioneer in HD research](#) and a member of the team that discovered the huntingtin gene in 1993. Dr. MacDonald presented the latest data on so-called modifier genes, which can affect disease onset by decades.

In the past, I've only ever been able to just say hello to Dr. MacDonald. However, during a free moment after her talk, I told of the research's importance for my own life: it may explain why, with the same level of defect in my HD gene as my mother, I have gone a decade without symptoms beyond her apparent age of onset.

I told Dr. MacDonald that the discovery of modifier genes – and the more precise prediction of onset – might open up a new round of genetic testing for the HD community, although I added that I wasn't sure I wanted to go through the difficult experience of testing again.

Later, at the farewell dinner, I hugged and thanked Dr. MacDonald for her work, which, as [Dr. Pacifici noted](#), could lead to drugs mimicking the actions of the modifier genes. (Also [click here](#) to read more.)

Making a difference

I also interacted with a dozen advocates and family members. We discussed numerous HD-related matters.

For an upcoming article, I interviewed Scott Schobel, M.D., M.S., [Roche's](#) clinical science leader of product development and leader of its HD scientific team, for an update on the company's historic Phase 3 clinical trial to test the Ionis-Roche gene-silencing drug, which, if successful, could slow, halt, and perhaps even reverse HD symptoms. In the words of Roche personnel, they politely "turned the tables on me" by interviewing me on video about my advocacy and family's struggle against HD for a forthcoming awareness-building campaign.

Together, I feel we are making a difference in the fight against Huntington's disease!

The nonprofit CHDI is assisting immensely by providing funding, tools, guidance, and open-source data.



Above, HD advocates Jeff and Debbie Mulligan (seated) with (from left to right, standing) Frances Saldaña, HDSA CEO Louise Vetter and Gene Veritas (aka Kenneth P. Serbin) (photo by David Saldaña). Below, Janet Rafferty (in pink blouse), Roche's international communications leader for neuroscience and rare diseases, interviews Gene Veritas (photo by Charlotte Peterson, Edelman agency).



CHDI's 'Oscars' ceremony

As he does each year, HD global advocate and former NBC News foreign correspondent Charles Sabine – also a presymptomatic gene carrier – added his own dose of humor with his mini-version of the conference Oscars, “The Charles’.” Presented on the last evening, they’re a hit with the audience after three days of nonstop scientific panels.

A notable, quite appropriate honor resulted from the incorrect medical instructions given to keynoter Rod and his family prohibiting his mother from consuming pork, one of her favorite dishes. Thus, Charles stated, the “medical intervention of the conference award” went to man “who told our keynote speaker, Rod Man, that his mom can eat as much pork as she likes.”

The awardee was long-time HD specialist Mark Guttman, a neurologist at the Centre for Movement Disorders in Toronto, ON. ([Click here](#) to watch the awards program.)

A certain path ahead

Minutes later, on a more serious note during his closing remarks, Charles displayed the same [eloquence](#) exhibited during his introductory statement at the HD community’ historic meeting with Pope Francis at the Vatican in 2017.

In Palm Springs, Charles noted that, thanks to the advances of the scientists, the future path for patients and gene carriers is “no longer unremittingly downward.” This absolutely marks the existence of hope, he added.

To illustrate his point, Charles recounted his first experience at a recent [Huntington’s Disease Youth Organization](#) (known simply as HDYO) summer camp in the United Kingdom, with 65 young people.

“It was the hardest HD event of my life,” Charles said. He recalled how one distraught teen asked him: “Tell me something that you know for certain.”

In his ten years of travels around the world to meet with HD families, Charles had never heard a question so difficult to answer.

“A platitude wouldn’t suffice, nor a statement of which I was not one hundred percent certain,” Charles continued. “After a nod and a big breath, I replied: ‘No generation, yours included, will ever need to fear this disease as much as mine did.’ And the reason that I could say those words with such confidence was the tireless work and commitment of all of you in this room.

“So on behalf of all of those young people around the world, thank you.”

([Click here](#) to watch Charles. [Click here](#) for my video album of the 14th Therapeutics Conference.)



Charles Sabine at the HD Therapeutics Conference podium (photo by Gene Veritas)

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

Labels: [advocates](#) , [Charles Sabine](#) , [CHDI](#) , [depression](#) , [HD Therapeutics Conference](#) , [Huntington's disease](#) , [Marcy MacDonald](#) , [modifier gene](#) , [presymptomatic gene carrier](#) , [Roche](#) , [Rod "Rod Man" Thompson](#) , [symptoms](#) , [treatments](#)

No comments:

[Post a Comment](#)

[Newer Post](#)

[Home](#)

[Older Post](#)

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