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At Risk for Huntington's Disease

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## Riding the advocacy revolution: stem cell activists. the future of CIRM, and public awareness

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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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## HD Links

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

SATURDAY, JANUARY 25, 2014

## Riding the advocacy revolution: stem cell activists, the future of CIRM, and public awareness

If the promise of stem cells to treat a myriad of diseases – a potential outlined in my first of two reports on the [sixth World Stem Cell Summit](#) – is to be fulfilled, advocates and patient organizations must exercise effort and expertise in helping steer the research.

Starting with AIDS-related patient advocacy, a “revolution” has occurred over the past two decades in how patients have related to their doctors and the pharmaceutical industry, said Kevin McCormack, the senior director of Public Communications and Patient Advocate Outreach for the California Institute for Regenerative Medicine (CIRM). CIRM was one of the leading co-sponsors of the summit, held last month in San Diego.

“This is a really exciting time and really interesting time in medicine,” McCormack said, introducing a panel on patient advocacy and stem cell research on December 5. “There’s a lot of change going on. Part of it is due to all the advances that have been taking place, all the progress that is being made in many different fields, but obviously in stem cells in particular. That’s why we’re here.

“There’s also a change in the way we’re engaged. In the past it was a very paternalistic system, for want of a better word, where you went to the doctor, and the doctor said, ‘This is what you have,’ and then the doctor said, ‘This is how we’re going to treat you.’”

Now, said McCormack, that has changed. “Patients and patient advocates are really demanding more of a role, more of a voice, beginning with HIV-AIDS, where people just didn’t want to wait around ... to get the therapies,” he said. “And so they pushed and advocated and demanded in every part of the decision-making process.”

Beyond the key issue of Huntington’s disease advocacy, this article also addresses the future of the world-leading, public-bond-supported CIRM and the need for greater public education about the stem cell field, including safety concerns.

### Show up, get involved

Judy Roberson, RN, one of California’s leading HD activists, led off the above-mentioned panel with a simple but crucial tenet of advocacy: “Show up for things. Show up for meetings like this. Join support groups and national organizations.... When you get involved, opportunities come your way.”

Such opportunities become available because major “decision-makers” usually attend scientific meetings such as the Huntington’s Study Group conference she keynoted in 2009, Roberson pointed out.

[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](#)

As a result, Roberson, then the president of the Northern California Chapter of the [Huntington's Disease Society of America](#) (HDSA) and head of the Joseph P. Roberson Foundation (founded by and named for her HD-stricken brother-in-law), was invited by the Food and Drug Administration (FDA) to become an FDA HD advocate. The FDA oversees clinical trials for drugs and approves treatments. Roberson's husband Tim died of HD at the age of 51.

"You'll never get anything unless you ask for it," Roberson continued, recounting how in the early 1990s she approached her neighbor, California State Senator Mike Thompson, to seek support for Huntington's programs. Thompson, who headed the legislature's powerful budget committee, helped allocate \$1 million for HD programs over a period of five years. [Thompson](#) now serves in the U.S. Congress.

More recently, Roberson and other California HD advocates worked to help Drs. Vicki Wheelock and Jan Nolte of the University of California, Davis, secure a \$19 million grant to conduct research towards a stem cell trial for HD, as mentioned in my previous article on the summit and detailed in a [2012 article](#).

You can watch the Stem Cell Summit advocacy panel, including Roberson's speech, in the video below.



### How Patients and Patient Organizations Are Impacting Advances in Stem Cell Research and Regenerative Medicine

from [Gene Veritas](#)

58:32 |

[How Patients and Patient Organizations Are Impacting Advances in Stem Cell Research and Regenerative Medicine](#) from [Gene Veritas](#) on [Vimeo](#).

#### An eye-opening experience

For Katie Jackson, also of Sacramento and the vice president for [Help4HD-International](#), a newly emerging patient and family support organization, the summit opened up a new world. Jackson's husband was diagnosed with HD in 2006. The couple has two untested children with a 50-50 chance of inheriting the HD mutation.

"This summit has been so eye-opening and so amazing," Jackson told me in an interview on December 5. "I thank the California Institute for Regenerative Medicine for sending me here.... From the second you wake up in the morning till the second you go to bed, you're amongst these innovator-researchers that are just changing the world."

Jackson said she was excited to be "part of the medicine that's today, and that is regenerative medicine.... People are starting to understand that this is important stuff."

The Stem Cell Summit left Jackson with a feeling of great hope, she said.

“There are a lot of people that are working hard to find therapies and possibly the cure,” she said. “Hopefully this is the last generation of Huntington’s disease.”

You can watch the entirety of our interview in the video below.



## Huntington's Disease Advocate Katie Jackson at the World Stem Cell Summit: An Interview with Gene Veritas

from [Gene Veritas](#)

12:25 |

[Huntington' Disease Advocate Katie Jackson at the World Stem Cell Summit: An Interview with Gene Veritas](#) from [Gene Veritas](#) on [Vimeo](#).

### **The center of the stem cell universe**

As stated by outgoing CIRM President Dr. Alan Trounson in his [keynote speech](#) on December 4, numerous CIRM-supported projects are putting the drive towards stem cell treatments into “high gear.”

According to Jonathan Thomas, the chair of the Independent Citizens’ Oversight Committee (ICOC), CIRM’s governing board, the agency is currently funding research regarding 40 incurable diseases and conditions. That research is part of projects funded at 70 different California universities, research institutions, and biotech companies.

Critically, these efforts aim to get potential therapies into human clinical trials, the final, crucial step before the FDA can approve a drug.

CIRM, approved in a 2004 statewide vote on Proposition 71, has so far spent \$1.85 billion of its mandated \$3 billion budget, funded by state-issued bonds.

California was already “loaded” with research talent, Thomas observed in a CIRM public forum on December 3, “but the fact that CIRM is here has enabled the state to attract senior stem cell scientists from all over the world just to have the opportunity to get funded for their work.”

Thanks to CIRM, California has become “the center of the stem cell universe” and the “envy of the rest of the world because of what the voters have enabled us to do,” Thomas continued.

“No other state has been able to duplicate this,” he said. “There are smaller efforts that are funded either by annual appropriations by state legislatures or largely funded through philanthropic gifts or whatever. No place has the bonding authority we do.”

### **Keeping the research pipeline open**

CIRM will fund its final round of projects by 2017. Actual administration of the grants will extend to about 2021, McCormack said.

“But obviously the key question here is: what about funding the research that’s already in the pipeline, about the kind of exciting progress that we’ve made that we don’t want to see wither and die, because if we go away, who’s going to fund it?” he said.

McCormack explained that continued support for the early and middle stages of a project enables the researchers to avoid the so-called “valley of death” – a lack of funds preventing the scientists from approaching large drug companies to promote the idea of the large, expensive, and sometimes lengthy Phase III clinical trials, the final stage before FDA approval.

“We’re looking at a number of different ways of getting new money – private funding, philanthropy, a number of different choices that we’ll be exploring,” McCormack said. “We’re not ready to go public yet with some of the things we’re looking at, but hopefully we’ll be able to find some way of continuing this research.”

“I hope with all my heart that we can go for a part 2 of Proposition 71,” said panel member Don Reed, a leader in the 2004 effort, who became active in the cause because of his son Roman’s paralysis resulting from a college football accident in 1994. “It’s going to be the greatest stem cell battle in the history of the world, and if we win, there’s just going to be unbelievable stuff that will happen.... Support it. Send letters to the editor. Fight every we you can.”

Roberson noted that Prop 71 originator Bob Klein’s comments at the Stem Cell Summit included plans for an “advocate boot camp” in support of CIRM. Klein served as the first chair of the ICOC from 2004-2011.

Panel member Alex Richmond, an advocate for treatment of children’s neurological disorders and the executive director of Children’s Neurobiological Solutions, observed that, as a public agency, CIRM cannot advocate for its own existence. “So it puts more pressure on organizations like ours and individuals like you, individual scientists that are out there, to be really as good an advocate as they can,” he said.

### **Public education critical**

Researchers speaking at the CIRM public forum and presenting projects at the Stem Cell Summit both emphasized the need for greater public understanding of stem cell science.

In addition to reports on specific diseases such as Parkinson’s, AIDS, and cancer, the public forum provided easily understandable introductions to stem cell basics, focusing on such themes as adult stem cells, pluripotent stem cells (which can become any kind of cell type), and cell replacement therapy.

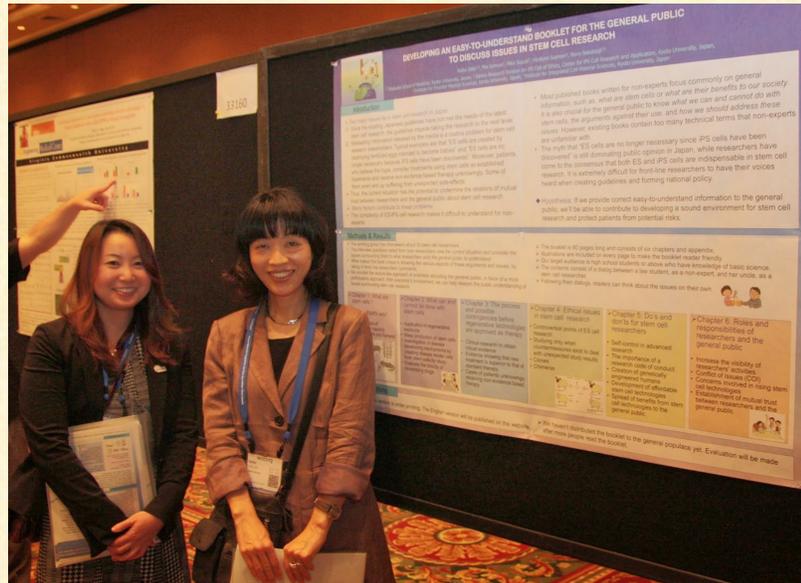
You can watch videos of all the public forum presentations, as well as a number of other panels and presentations of the summit, by visiting my [2013 World Stem Cell Summit video album](#), to which I’ve just added twelve more presentations.

To promote stem cell education, two researchers presented a poster titled “Developing an Easy-to-Understand Booklet for the General Public to Discuss Issues in Stem Cell Research.” Authored by Keiko Sato, Ph.D., and researcher Mie Samura of Kyoto University, Japan, the project seeks to correct misinformation and misunderstanding about the field.

“Misleading information released by the media is a routine problem for stem cell research stakeholders,” they stated on their poster, which refers

to Japan but can be applied to the U.S. and other cultures. They cited the examples of embryonic stem cells, reported in some media outlets as being “created by destroying fertilized eggs intended to become babies.”

The book will appear in Japanese and also online in English.



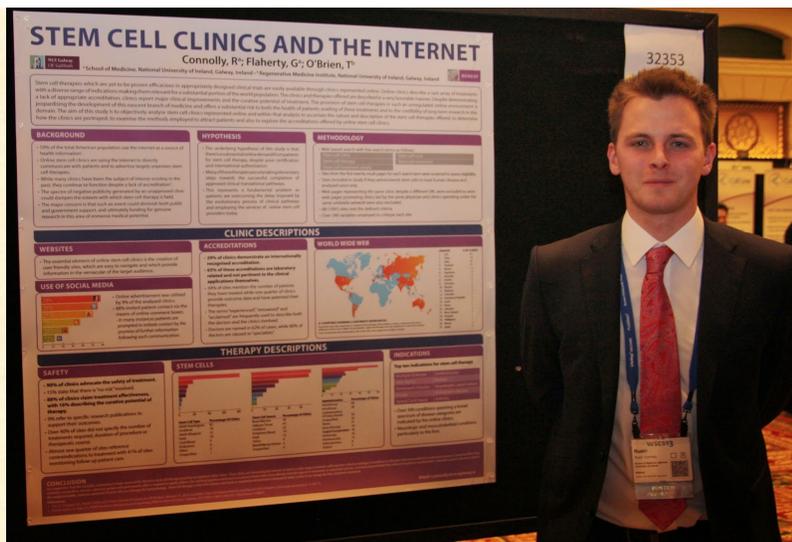
*Dr. Keiko Sato (right) and researcher Mie Samura at their poster about stem cell education (photo by Gene Veritas)*

Their poster also warned about the “hype” surrounding unproven stem cell treatments, sometimes leading to “unexpected side effects” in patients who try them.

“Thus, the current situation has the potential to undermine the relations of mutual trust between researchers and the general public about stem cell research,” the authors wrote.

The poster “Stem Cell Clinics and the Internet,” by Dr. Ruairi Connolly and two collaborators from the National University of Ireland, further underscored the dangers of unproven treatments offered by online clinics.

“Despite a lack of appropriate accreditation, (such) clinics report major clinical improvements and the curative potential of treatment,” they wrote. “The provision of stem cell therapies in such an unregulated online environment is jeopardising the development of this nascent branch of medicine and offers a substantial risk to both the health of patients availing of these treatments and to the credibility of long term research in this domain.”



Dr. Ruairi Connolly with poster about unaccredited, online stem cell clinics (photo by Gene Veritas)

(Disclaimer: I received a stem cell summit scholarship from CIRM, which covered the cost of registration. CIRM officials did not in any way influence or control what I have written here.)

Posted by [Gene Veritas](#) at 1:23 PM

Labels: [advocacy](#) , [awareness](#) , [California](#) , [CIRM](#) , [Huntington's disease](#) , [Jonathan Thomas](#) , [Judy Roberson](#) , [Katie Jackson](#) , [regenerative medicine](#) , [revolution](#) , [stem cells](#) , [treatments](#) , [World Stem Cell Summit](#)

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