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Advocacy pays off: Huntington's disease stem-cell research advances in California

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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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MONDAY, JANUARY 23, 2012

Advocacy pays off: Huntington's disease stem-cell research advances in California

Grass-roots advocacy for Huntington's disease stem-cell research is paying off.

Using funds from the state of California secured with the help of advocates, leading HD stem-cell scientists are taking important steps towards developing potential ways to treat, reverse, and perhaps even cure HD.

These funds come from the [California Institute for Regenerative Medicine \(CIRM\)](#), the \$3 billion initiative approved by the state's voters in November 2004 to establish grants and loans for stem-cell research aimed at rapidly developing treatments for a host of diseases.

Leading stem-cell advocates had successfully worked to get the measure on the ballot as a state initiative after President George W. Bush had signed an order blocking the establishment of new human embryonic stem-cell lines, which scientists needed to expand research in the field. President Barack Obama later rescinded that order.

CIRM projects have spurred the creation of new embryonic stem-cell research, as well as other kinds of stem cells.

Millions in funding

While the CIRM oversight board has representatives concerned with Alzheimer's disease and diabetes, among other conditions, it has no representative from the Huntington's disease community.

Nevertheless, in 2007, as CIRM prepared to award its first research grants, California's grass-roots HD activists began campaigning for the board to address Huntington's stem-cell projects.

In October of that year, I arranged for Bill Johnston, the public relations director of the San Diego Chargers and the president of the San Diego Chapter of the Huntington's Disease Society of America, to appeal to the oversight board during a public meeting in San Diego. As he spoke, Johnston held his wife Ramona, who has HD. This was the very first time that a member of the HD community had appeared before the board.

I also spearheaded the organization of the December 2007 CIRM "Spotlight on Huntington's Disease," held at the University of California, Los Angeles. The presentation included talks by two leading scientists, as well as a plea for HD research from activist Frances Saldaña and her daughter Margie Hayes, one of three siblings who developed juvenile HD and the mother of two at-risk children. (For more on these first meetings, [click here](#).)

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 GENE VERITAS

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As mother Frances Saldaña (left) looks on, Margie Hayes tells about her struggle against HD at the CIRM Spotlight on Huntington's Disease, Los Angeles, December 12, 2007 (photo by Gene Veritas).



CIRM President Dr. Alan Trounson (left) with Alexa Shaffer and mother Sharon. Sharon has HD, and Alexa is at risk. They told their stories at a CIRM board meeting in San Diego on September 25, 2008 (photo by Gene Veritas).

Advocates throughout California joined hands in this cause, with other HD family members speaking before the oversight board at its regular public meetings in different parts of the state. (For the sake of brevity, I won't list the names of the numerous dedicated individuals involved in these efforts.)

Together with the researchers' meticulously prepared grant applications, this advocacy has generated solid results: to date, HD stem-cell research projects, based mainly at the University of California, Davis (UC Davis), and the University of California, Irvine (UCI), have received \$7.9 million in CIRM funding.

Striving for an HD 'Disease Team'

Tomorrow, January 24, Jan Nolte, Ph.D., the director of the UC Davis Institute for Regenerative Cures, will submit an application to CIRM for a \$20 million project to fund an HD "Disease Team." The team will aim to develop the very first human clinical trial for an HD stem-cell treatment,

which would involve attacking the disease at its genetic roots and infusing the brain with an important growth factor known as [BDNF](#).

Just last month, Dr. Nolta published a scientific article demonstrating promising results in pre-clinical experiments, which must precede human trials ([click here](#) to read more).

Dr. Nolta uses a well-known type of stem cells, called “mesenchymal stem cells.” A long-time expert on these cells, Dr. Nolta refers to them as “paramedics” because of the way they congregate around and repair damaged cells.



Dr. Nolta at the HD bench at the Institute for Regenerative Cures (photo by Gene Veritas)

Last May I spent a day interviewing Dr. Nolta and observing the work of the UC Davis institute, co-founded by the university and CIRM ([click here](#) to read more about my visit and Dr. Nolta's work).

Once again, we will mobilize our California network of advocates, this time in support of the HD Disease Team application. We want to stress the urgency of finding treatments for this killer disease and the importance of HD research in advancing the stem-cell field.

New stem-cell lines

At UCI, HD stem-cell research has progressed rapidly under the leadership of Dr. Leslie Thompson, a professor in the UCI School of Medicine's Departments of Psychiatry and Human Behavior, the interim director of the Center for Mitochondrial and Molecular Medicine and Genetics, and a holder of various other positions within the university.

With one CIRM grant of \$900,000, awarded in 2008, Dr. Thompson and her researchers are creating new neuronal (brain) stem cells. They produce these cells by taking skin-cell samples from both HD-affected and non-HD people, “reprogramming” them to become stem cells, and then “differentiating” them into the neuronal stem cells.

From these new cells, they eventually hope to obtain medium spiny neurons, the kind of brain cell most affected by HD. The disease occurs because these cells malfunction, become damaged, and die. Using the neuronal stem cells, the research team can study HD at work in a live, real-time human setting.

This grant also funds research on human stem cells taken from embryos discarded by couples who underwent PGD (preimplantation genetic diagnosis) in order to have HD-free children. In this project, Dr. Thompson and her researchers have succeeded in establishing one new line of human embryonic stem cells, which will be used to study HD.

Announced in late 2010, a second CIRM grant of \$3.8 million funds a UCI project that seeks to develop a stem-cell treatment for study in HD mice. The initial experiments in this project demonstrate that the mice's symptoms improve after the introduction of mouse stem cells into their brains. The second stage is examining the effect of human stem cells on the mouse brains.

Later, Dr. Thompson hopes to apply for another HD Disease Team grant to develop ways to apply her research for potential stem-cell treatments in humans.

In future articles, I will explore in greater depth how Dr. Nolta's and Dr. Thompson's respective projects could lead to effective treatments.

The impact of our work

Interviewing Dr. Thompson in her office on January 20, I felt a deep sense of accomplishment.

In an instant, I felt as if time had fast-forwarded me from 2007, when she and I prepared intensely to organize the CIRM Spotlight on HD, to the present, when the results of the statewide advocacy efforts are blooming in the UCI labs.

I had advocated *with* and *for* Dr. Thompson, and now I was sitting across from her and hearing good news.



Dr. Thompson (left) with Frances Saldaña at the Huntington's Study Group meeting in San Diego, October 16, 2010 (photo by Gene Veritas)

In voting for the CIRM, and then advocating for specific HD projects, we had achieved a historic breakthrough for HD research.

In hearing Dr. Thompson discuss the establishment of a new stem-cell line from the PGD embryos, I felt how profoundly *political* our advocacy has been, and how it directly impacts the quest for treatments and a cure.

The moment was exhilarating.

Gene Veritas: Back when the big controversy was occurring, when Bush said, "No more new lines," this is a --

Leslie Thompson: A new line.

GV: This is a new line that, thanks to the CIRM and the law in California, was supposed to occur.

LT: Thanks to the CIRM. Correct.

GV: So we're seeing an actual impact of a political decision here to do something.

LT: Yes. All of it. All of [the stem-cell work]. Because we wouldn't be able to do this work without CIRM funding.

We can all advocate for the cause

Advocacy is one of the biggest challenges for the HD movement. It demands long hours of preparation, the study of complex issues, networking, and efforts to gain access to public officials and other powerful individuals.

It also requires patience, dedication, and teamwork. We California advocates have recognized these requirements and tried our best to practice them.

On the stem-cell front, we have in our favor a great set of universities with top-flight scientists like Dr. Nolta and Dr. Thompson. Both are well-known in the HD community, and both dedicate time outside of work to supporting it. Dr. Thompson finds inspiration in HD families. A newcomer to HD research, Dr. Nolta took on this new field after coming into contact with the local HD medical specialist, Dr. Vicki Wheelock, and the community she serves.

Advocates and scientists have meshed well throughout the crusade for stem-cell treatments.

Finally, I believe our advocacy has proved effective because of the willingness of affected families to tell their stories in public – despite the pain or awkwardness it might cause – at CIRM meetings and to the doctors and physicians involved in HD research.

All of us in the HD community can become advocates. We need people to work on all levels – from meeting with government officials to writing letters. And we must always speak out.

You never know. Someone with the willingness and resources to help might just be listening. *You* can inspire that person to act.

(To learn more about California HD advocacy, please [click here](#). To learn about national advocacy efforts, please [click here](#).)

Posted by [Gene Veritas](#) at 9:51 AM     

Labels: [advocacy](#) , [advocate](#) , [brain](#) , [California](#) , [CIRM](#) , [clinical trial](#) , [cure](#) , [Huntington's](#) , [Jan Nolta](#) , [Leslie Thompson](#) , [medium spiny neuron](#) , [mesenchymal stem cell](#) , [PGD](#) , [stem cell](#) , [symptoms](#) , [treatments](#)

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