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Stem cells: the dawn of a new era for Huntington's disease research

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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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SATURDAY, DECEMBER 29, 2007

Stem cells: the dawn of a new era for Huntington's disease research

The campaign for stem-cell research on Huntington's disease took an important step forward on December 12.

The 29 members of the governing board of California's $3 billion stem-cell institute heard from two leading HD researchers about the large potential of stem-cell research in developing treatments for HD. The presentations took place at the board's monthly meeting, held at the University of California, Los Angeles. These influential leaders from academia, the bio-technology industry, and patient advocate groups will decide in the coming months whether a proposed California HD "disease team" will receive a $50,000 planning grant and potentially millions of dollars for an intensive, statewide effort to attack HD using stem cells.

A historic moment

For nearly an hour the Independent Citizens' Oversight Committee (ICOC) of the California Institute for Regenerative Medicine (CIRM) watched a detailed presentation by Dr. Robert Pacifici, the chief scientific officer of the California-based Cure Huntington's Disease Initiative, Inc. (CHDI), Dr. Hans Keirstead, a stem-cell dynamo at the University of California, Irvine, and HD activist Frances Saldaña of Orange County and her daughter Margie Hayes, one of three siblings who developed juvenile HD and the mother of two at-risk children.

Working with my fellow activists in the San Diego chapter of the Huntington's Disease Society of America (HDSA), the Orange County affiliate, and the Los Angeles chapter, I spearheaded the organization of the CIRM's "Spotlight on Huntington's Disease." Like previous CIRM spotlights on other diseases, this event was open to the public. I joined HD researchers, activists, and families from throughout southern California to provide support and witness this historic moment in Huntington's disease advocacy.

HD takes center stage

I was especially heartened to see HD take center stage in California and hear the scientists' optimism about stem cells for HD research. Many of us in the HD community have felt intuitively that stem cells are crucial in the search for a cure and should receive emphasis immediately. At the CIRM meeting I had the strong sensation of being fast-forwarded into a future where HD will no longer threaten the lives of people like Margie, who, her speech impeded and her body dancing uncontrollably, moved ICOC chair Robert Klein and the rest of the audience with her sad story of testing positive for HD, suffering discrimination, and becoming incapacitated.

CHDI efforts

Dr. Pacifici, whose non-profit CHDI has stepped in where the big drug

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companies have failed to venture, stressed the importance of stem cells in recreating the actual human version of the disease in a Petri dish. CHDI, he explained, has set up some twenty parallel programs aimed at finding treatments.

"Without exception there isn't a program in our portfolio that wouldn't benefit dramatically from having a stem-cell-derived cell population in its flow scheme," he stated. HD stem cells would enable researchers to understand such puzzles as the differing ages of onset in those individuals with identical genetic defects (the so-called CAG repeats of Huntington's disease) and whether brain cells are dying as a result of "murder or suicide."

Significantly, stem cells would speed up the process of screening for drugs, Pacifici said.

New hope

Pacifici then offered the kind of hope that I could only dream of as my own mother withered away and ultimately died because of Huntington's nearly two years ago – a hope that I can now cling to as I approach my own inevitable symptoms. The same cocktail of compounds used to turn stem cells into brain cells in a Petri dish, Pacifici stated, could be used as drugs to repopulate damaged areas of the brain with new cells. "It may be that we can change our aspiration from preventing or slowing Huntington's, to actually reversing Huntington's disease, if the promise of regenerative medicines and cell-based therapies comes to fruition," he said.

Dr. Keirstead underscored the urgency of using stem cells in HD research and detailed how the hope can become reality.

"I was an absolute convert to HD," said Keirstead of the request by UC Irvine colleague Dr. Leslie Thompson that he ply his internationally recognized stem-cell expertise in the area of Huntington's. "Huntington's disease is truly, in my opinion, one of the few diseases that's applicable in the short-term" to two key stem-cell research strategies – using cells to understand how the disease works and to actually treat people. HD's character as a disease with a single, discovered genetic cause and its impact on specific areas of the brain make it one of those unique candidates for stem-cell research, he explained.

Little scientific attention

Despite this enormous potential, HD stem-cell research has received little attention from the scientific community.

"It absolutely amazes me that there is not a handful – not one – human embryonic stem-cell line of HD.... Not a one in the entire world," Keirstead stated, referring to the lack of scholarly articles on stem cells derived from human HD embryos. "That's just remarkable. With the technologies of deriving embryonic stem cells from blastocysts [the human before it becomes an embryo], with the adoption of prenatal genetic diagnosis by many HD-bearing couples, it amazes me that we don't yet have a stem-cell line or two or three or four or thirty or three hundred that bear this mutation, a mutation of a gene that we absolutely know of and that we have access to.... Yet we know precisely how to obtain those things. We know very routinely how to make them.... This is going to be one of the major, major tools."

Keirstead and UCI are wasting no time in taking the lead. "We are deriving new lines at UCI," Keirstead explained. "I have recently established relationships with fertility clinics. I now have access to over 10,000 blastocysts a month. That's phenomenal.... We are already receiving phone calls from people with Huntington's disease that have [gone to] fertility clinics that have HD-positive blastocysts that they would like to channel

our way. I have about fourteen such individuals so far with the three fertility clinics that I am working with."

Thinking long-term

Keirstead is a strategic thinker who sees the long-term implications of his work.

"You can't walk into the FDA – two guys and a rat – and say, 'Let me try this in humans,'" he said. Keirstead transforms stem cells into other cells in a manner compliant with the standards of the Food and Drug Administration and in quantities large enough to make their use commercially viable for large biotech companies. He deals in not just "a few cells in a dish," but in the billions. Only in this way, he explained, will a company invest the many millions of dollars necessary to getting the stem-cell process to treatments. Keirstead's work includes pushing stem cells to become neurons for the striatum, the area of the brain devastated by Huntington's disease.

"I don't think we should lose sight of the power of this. If we can actually make high-purity populations of striatal interneurons, what's going to happen to this field is precisely what's going to happen to the spinal cord field, which is: a commercial entity will come in and value-add, that more researchers will take those cells... and begin working on them in high-purity format. And we're going to see a turning point. It's the dawn of a new era for HD."

Increased advocacy role

I hope that the CIRM spotlight on HD was also a new dawn for HD advocacy and Californians' central role in it. In chairman Klein's words, "The Huntington's organization's patient advocacy is extraordinary. I have tremendous respect for the passion, intensity, tenacity, and commitment of the Huntington's organization." Klein had earlier noted that HDSA was the only disease advocacy group to have done presentations in both the CIRM "spotlight" format and in the public hearing of the ICOC board. The previous presentation took place at the October 3 CIRM meeting in San Diego, where HDSA-San Diego president and San Diego Chargers' public relations director Bill Johnston and his wife Ramona, who has HD, appealed to the board to support HD research.

The December 12 spotlight came just weeks after CHDI announced another stunning new project in HD research to take place in southern California. On October 26 CHDI and Carlsbad-based Isis Pharmaceuticals, Inc. revealed that they would collaborate in a multi-million-dollar effort to develop a drug to target directly the huntingtin gene, the cause of Huntington's disease. I will write about this exciting development in a future entry.

Posted by Gene Veritas at 2:52 PM

2 comments:

Anonymous said...

Hi- you write about there being no stem cell lines for HD. My husband and I have persnally donated for science our remaining embryos after PDG. I am wondering if you have any recommendations on how I can follow up on this to understand what happen to them and if we decide to do PGD again, where should we go to donate?

reneel72@comcast.net

10:03 AM, January 18, 2008

Angela_F said...

Oooh I'd be interested in doing that, we're going for PGD in the next few months. Will send you an email to ask about that though.

9:04 AM, April 23, 2008

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