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California stem cell agency approves $19 million clinical trial project as Huntington's disease families 'change the course of science'

Kenneth P. Serbin
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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.

Blog Archive

- ► 2021 (12)
- ► 2020 (16)
- ► 2019 (19)
- ► 2018 (16)
- ► 2017 (14)
- ► 2016 (13)
- ► 2015 (24)
- ► 2014 (24)
- ► 2013 (30)
- ▼ 2012 (26)
  - ► December (2)
  - ► November (3)
  - ► October (1)
  - ► September (2)
  - ▼ August (2)
    - Playing in the fourth quarter of life as Huntingto...
    - California stem cell agency approves $19 million c...
  - ► June (2)
  - ► May (1)
  - ► April (2)
  - ► March (3)
  - ► February (3)
  - ► January (5)
- ► 2011 (33)
- ► 2010 (26)
- ► 2009 (21)
- ► 2008 (7)
- ► 2007 (7)
- ► 2006 (4)
- ► 2005 (17)

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FRIDAY, AUGUST 03, 2012

California stem cell agency approves $19 million clinical trial project as Huntington's disease families 'change the course of science'

Adult stem cells designed to rescue brain cells from death in Huntington's disease patients could enter human testing in the next three to four years, thanks to a $19 million grant to an HD research team at the University of California, Davis (UC Davis), from the California Institute for Regenerative Medicine (CIRM).

If successful, this first-ever stem cell clinical trial for Huntington's could pave the way for a possible treatment of the devastating disorder.

At a public meeting July 26, the oversight board of the $3 billion stem cell agency announced the award to the lab of researcher Jan Nolta, Ph.D., a recognized specialist in mesenchymal (pronounced "meh-zen-KI-mal") stem cells (MSC), and her collaborator Vicki Wheelock, M.D., a neurologist and the director of the Huntington's Disease Society of America's Center for Excellence for Family Services and Research at UC Davis.

Dr. Nolta aims to introduce MSCs, which act as natural "paramedics" in the body, into the brains of symptomatic HD patients to test for safety and tolerability. The trial doses will be made from a sample of MSCs extracted from a healthy donor.

MSCs produce a so-called "fertilizer for the brain" (BDNF, brain-derived neurotrophic factor), whose levels plummet drastically when someone has HD. Dr. Nolta and her team have engineered MSCs to produce higher levels of BDNF in an attempt to help HD-damaged neurons recover and avoid death, thus slowing, halting, or perhaps even reversing the course of HD.

Dr. Nolta's collaborator Gary Dunbar, Ph.D., of Central Michigan University, has already demonstrated that these MSCs mostly stop symptoms in transgenic mice that have been given the abnormal HD gene.

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Dr. Jan Nolta (above) at the HD work bench at the Institute for Regenerative Cures. Below, Dr. Vicki Wheelock (photos by Gene Veritas).

The Nolta-Wheelock grant was one of eight CIRM grants totaling $151 million to labs seeking treatments for debilitating or fatal diseases, including Lou Gehrig's disease, cancer, heart disease, and spinal cord injuries. The awards were the second largest research round in CIRM history. In 2009 the agency granted more than $200 million to researchers.

With a score of 87/100, the Nolta-Wheelock grant ranked highest in the state.

"We're just so glad that we didn't let the community down," Dr. Nolta told HD activist Melissa Biliardi on *The HD View* internet radio program on July 23 in anticipation of the expected award.

In this same round UC Davis received two other grants – to seek treatments for peripheral artery disease and osteoporosis – that Dr. Nolta will help oversee in her role as the director of the UC Davis stem cell program and the university's Institute for Regenerative Cures (IRC), which has nearly 150 affiliated faculty researchers.

"People are hopeful, truly hopeful for the first time," Judy Roberson, the former president of the Northern California Chapter of the Huntington's Disease Society of America (HDSA) and the widow of an HD victim, said after the CIRM announcement. "This is a nightmarish, cruel disease in every way but now, thanks to CIRM, we are turning the dream of a stem cell therapy trial into a reality. Research means hope for people with this disease, but research costs money. CIRM has given us all hope."

The trial's proposed timeline

CIRM will grant the $19 million over four years, the proposed timeline of the clinical trial project. Most of the money will cover charges such as surgeries, operating room and hospital costs, MRI scans, and other items related to the actual trial.

According to the proposal, the UC Davis team will spend the first year testing the safety of MSCs in healthy non-human primates. This stage of the project will help the team secure the necessary approval for human testing from the U.S. Food and Drug Administration (FDA), which regulates clinical trials.

In the project's second year the team hopes to enroll at least 26 early-stage HD patients in an observational study, including motor and psychiatric tests and MRI brain scans, to obtain basic measurements of their health for comparison with readings to be taken during the clinical trial.

At the start of the third year, if all regulatory approvals have been obtained as planned, the patients will receive a single, direct injection of the MSCs into each side of their brains (a bilateral intrastriatal injection). A special neurosurgical team, which will include experts from the University of California, San Francisco, will bore a tiny hole into the skull to insert a tiny cathether to deliver the cells. Direct insertion is necessary because of the blood/brain barrier, which allows few medications to enter the brain. Patients will have part of their heads shaved. However, their hair should grow back, and the holes will heal over.

Half of the patients will receive MSCs with the extra BDNF-producing capability, while the other half will receive a placebo, MSCs without that capability.

Trial participants will receive dosages in groups and on a staggered schedule, with each successive group receiving a higher amount of the MSCs.

The remainder of the trial will primarily check for the safety of the MSCs. As a secondary goal, the scientists and physicians will also look for alleviation of symptoms and evidence that the MSCs are improving the health of the brain.

This first step in the trial is known as Phase I. If the MSCs prove safe, the team would seek funding for Phases II and III to fully measure the cells' efficacy.

All of these plans must receive formal approval from UC Davis's internal review board and then the FDA, after which full details will become available for potential trial participants.

A brief history of stem cells

To understand Dr. Nolta's work we must travel back in time to explore the roots of today's revolution in stem cell research.

Stem cells became a hot topic in the first decade of the 21st century because of the controversy over one type: embryonic stem cells. However, stem cell research long predates this controversy.

Recall that a stem cell has a very important property: it can make cells that eventually become another type of cell such as a muscle cell, skin cell, or brain cell (neuron).

Stem cells help our bodies regenerate lost or worn tissue and components such as our blood, liver, and skin.

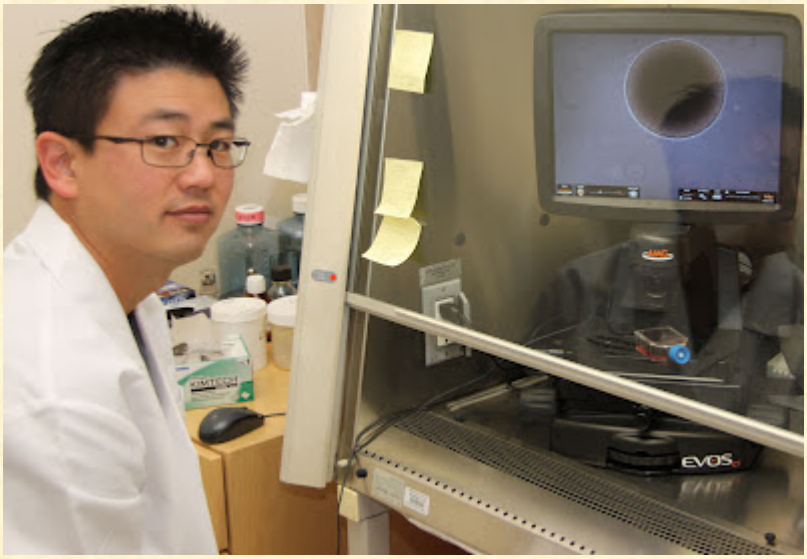
Humans have understood the idea of regeneration since ancient times, and scientists first started discussing the concept of stem cells in the mid-1800s. Scientists first discovered stem cells in mice bone marrow in the early 1960s.

The very first stem cell therapy (treatment) in humans took place in 1968 with the successful bone marrow transplant for a leukemia patient whose marrow donor was an identical twin. This type of transplant helps the patient because bone marrow contains stem cells that produce new blood cells. Because of stem cell research, other kinds of transplantation and tissue regeneration have become possible.

Over the last few decades, scientists have identified other types of stem cells, including those that produce neurons. Stem cell research is now burgeoning around the world. Scientists use stem cells both to understand human biology and to seek therapies for diseases and traumas.

In August 2001, President George W. Bush stopped federal funding for new embryonic stem cell research because of his belief, shared by a good number of Americans, that such research destroyed human life (the embryo from which the stem cells were taken) and was therefore immoral. In California Bush's restrictions spurred a successful movement to pass a 2004 ballot initiative, Proposition 71, that skirted the president's order with state-level funding, created CIRM, and catapulted the state into global leadership in stem cell research.

In recent years, however, new discoveries have lessened the controversy about stem cells. Scientists have made many advances using *adult* stem cells – those extracted from a living human being without any risk. In 2006 researchers achieved another milestone that reduced the need for embryonic stem cells: they could now take cells from the skin or other parts of the body and reprogram them into a stem cell.



Dr. Alvin King of the University of California, Irvine, displays a neural stem cell on the screen of a microscope (photo by Gene Veritas).

The MSCs, Dr. Nolta's focus for the past 25 years, are adult stem cells. Everyone has MSCs. They are found in the bone marrow, as well as in fat, dental tissue, and the umbilical cord. They can make bone, tendons, ligaments, and other connective tissues. MSCs grow well in lab conditions, making them a prime candidate for research.

Along with other scientists, in recent years Dr. Nolta and Leslie Thompson, Ph.D., of the University of California, Irvine, another CIRM grantee, began employing stem cells in Huntington's research. Besides MSCs, HD researchers use human embryonic stem cells, human induced pluripotent stem cells, neural stem cells, and others.

In Dr. Nolta's assessment, MSCs appear to have especially great potential in treating HD because of their abilities as the body's "paramedics." This potential is described in detail below.

From child scientist to MSC expert

Dr. Nolta's path to the potentially historic MSC HD clinical trial began in childhood and took shape in the midst of the stem cell revolution.

"I think I was probably born a scientist," she told me during a May 2011 visit to her lab on the occasion of the HDSA

Northern California Chapter's annual convention. "I was the kid that was out in the yard investigating bugs and watching eggs hatch and feeding baby animals that were rescued and trying to understand how caterpillars went through the chrysalis form and came out as moths and butterflies."

Raised by a single working mom in the small northern California town of Willows and depending on grants and waitressing for her college education, Dr. Nolta received a degree in biology from Sacramento State University in 1984.

After graduation Dr. Nolta took M.A.-level science courses at UC Davis and volunteered in a lab. "We could take stem cells from the bone marrow and culture them," she recalled. "There was this 'magical' potion that we could put them in and culture them for just a few days and could watch them divide and grow into blood cells. I wanted to secretly keep the cultures growing and study them.

"Where I fell in love with mesenchymal stem cells was in 1987. We started doing long-term bone marrow cultures, and there's a component that grows out when you take a marrow aspirate from a human being that's a mono-layer of broad, flat cells. We used to call those the marrow-stromal cells. They later got renamed to mesenchymal stem cells due to their potentiality and all that they can do."

Dr. Nolta learned that MSCs could assist greatly in gene therapy. Also known as cellular therapy, gene therapy involves the use or alteration of genes to treat disease. Dr. Nolta was impressed with MSCs' strong ability to assimilate and deliver gene therapy products.

"I realized very quickly that we could engineer them to even better support the other cells in the body," she explained.

To deepen her knowledge of stem cells and MSCs, Dr. Nolta enrolled in the Ph.D. program in molecular microbiology at the University of Southern California under the mentorship of Dr. Donald Kohn, a specialist in pediatric bone marrow transplantation. At Children's Hospital Los Angeles she assisted in his pioneering work on bubble baby syndrome, AIDS, and other conditions.

From this experience Dr. Nolta learned the techniques of gene therapy, growing stem cells, and applying stem cell therapies in the clinic. With Dr. Kohn's team, she performed the first cord blood gene therapy trial for infants born with bubble baby syndrome, a type of serious immune deficiency.

In 2002 the Washington University School of Medicine in St. Louis, one of the nation's top medical schools, recruited Dr. Nolta to help build its programs in gene therapy and stem

cell research. There she continued her work on gene therapy and MSCs and collaborated with her close colleague Gerhard Bauer, Ph.D., in the establishment of a GMP (good manufacturing practice) facility, a highly advanced lab crucial for producing cell and gene therapies.

The power of grassroots advocacy

However, the future of stem cell research lay in California. In 2007 UC Davis lured Dr. Nolta back to her home state to direct its stem cell programs under the umbrella of the brand-new IRC, the Institute for Regenerative Cures. CIRM awarded UC Davis $21 million to construct the IRC and its state-of-the art GMP facility. UC Davis contributed $40 million to the project.

With little knowledge of Huntington's disease, Dr. Nolta had no plans to include it in her research program at the IRC when she was recruited.

Around the state, however, HD advocates were telling their stories of the desperate need for treatments at the public hearings of the CIRM oversight board. They pushed hard for the CIRM to back HD research.

UC Davis stem cell program manager Geralyn Annett (left), HD patient Sharon Shaffer, Alexa Shaffer, and Dr. Nolta advocating for HD research at a CIRM board meeting at UC San Diego in 2008 (photo by Gene Veritas)

During her recruitment trip to UC Davis, Dr. Nolta met Dr. Wheelock of the HDSA Center of Excellence.

"Have you ever considered using stem cells to treat Huntington's disease?" asked Dr. Wheelock as she rode with Dr. Nolta in an elevator.

"You know, for the last 20 years, I have been researching how to use stem cells to treat every part of the body except

the brain," Dr. Nolta responded, citing the critical hurdle of the blood/brain barrier.

"The families impacted by Huntington's disease are truly remarkable," Dr. Wheelock rejoined. "I'd love to introduce you to them."

That conversation spurred Dr. Nolta to take a scientific interest in HD. More importantly, meeting the families deeply moved her. She decided to act.

With initial financial backing from HD advocates from the Sacramento area and elsewhere, Dr. Nolta delved into a project to find a way to use MSCs to combat HD.

Dr. Nolta used her early findings to apply for a grant from CIRM. In 2009 the agency awarded her lab $2.7 million to study the use of genetically reengineered MSCs to block HD at its genetic roots, first in lab dishes, then in mice (explained below).

During our interview at the IRC, Dr. Nolta pointed to the photographs of HD advocates on her desk.

"They change the course of what scientists do," she said, breaking into tears. "My life was forever changed."

In all, local fundraising efforts have provided some $100,000 for Dr. Nolta's work. Donations have included $15,000 from the Deshalamar foundation and $40,000 from Team KJ, an Illinois initiative in support of Kara Jean Fleming, a 40-year-old HD patient. The Joseph P. Roberson Foundation, named for the deceased husband of Judy Roberson, has also supported Dr. Nolta's work. Many other donors, large and small, have also contributed.

Watching the paramedics in action

With the new $19 million CIRM grant – the largest in Dr. Nolta's career – she and the UC Davis hope to set their MSC research on the path to a treatment.

The MSCs' many attributes make them attractive for treating HD.

"They're very social," Dr. Nolta explained as she played a highly magnified video in which the MSCs appeared to swim and greet one another like people playing in a swimming pool. "They like to interact with other cells."

The MSCs also move around the body with great facility, Dr. Nolta added. They can project little tubes, called nanotubules, that tunnel into cells and inject them with

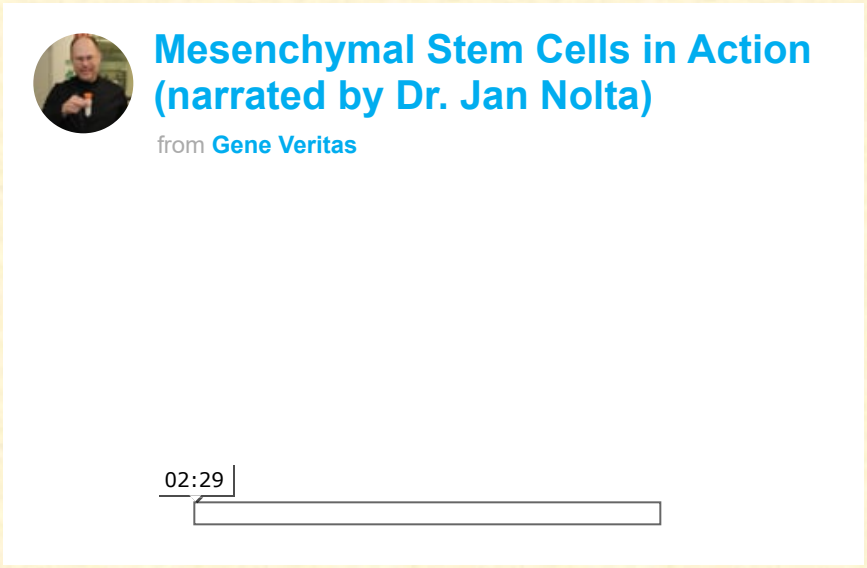
necessary items such as proteins and mitochondria, the powerhouses of the cell.

"It's like giving a cell new batteries," Dr. Nolta explained. "They just open up a nanotubule and put the new component into the other cell. So that's why we call them paramedics. It's like they're going around with tool kits to repair the other cells.... They like to check out other cells, to see if they're healthy. They can change what they produce from what they sense from the environment and from the other cells. They just become like little factories."

"They almost look like living organisms," I observed.

"They are," Dr. Nolta said. "They're alive."

(Watch the video below to see the MSCs in action.)



Mesenchymal Stem Cells in Action (narrated by Dr. Jan Nolta) from Gene Veritas on Vimeo.

The MSCs' sociability results in part from the fact that damaged or sick cells and neurons put out "distress signals" that spur the paramedics into action, Dr. Nolta continued.

The same process occurs in the brain, she added. In mice that carry the human Huntington's gene and have HD-like symptoms, MSCs injected into their brains migrated to the areas of damage.

Transplantations of human tissue often trigger a rejection by the immune systems of the recipients, requiring them to take anti-rejection drugs sometimes for the rest of their lives. This does *not* occur with MSCs, Dr. Nolta said.

"That's the beauty of them," she said. "They're transplanted from one patient to the next with really no regard to tissue matching. They actually shelter themselves from the immune system through some of the things that they secrete. We think that's part of their natural function in the body.

"When there's a wound or a heart attack or some kind of ischemic event, a stroke, they can go to that area, and they want to cause the tissue to heal without scarring. That's part of their innate mission. They don't want the immune system to see it while it's getting fixed up, because you could start making auto-antibodies to that damaged tissue, and then you would destroy that tissue. We think that the MSC just go to the scene of the injury and keep the immune system at bay while they're doing their remodeling. It's kind of like keeping everybody out of a construction site."

The goal: restoring neurons and connections

According to Dr. Nolta, the MSCs secrete substances that help restore the vital connections between neurons. Such connections are lost in HD. Additionally, in secreting BDNF and other brain growth factors, the MSCs can help damaged neurons recover. She likened this scenario to a chain of Christmas lights that, missing a bulb, will go out. Restoring the bulb – a healthy neuron – gets the whole chain working again.

In the case of the proposed clinical trial, the UC Davis team will ramp up the MSCs' capability to provide BDNF. In mice tests, they have increased that capability by a hundredfold.

The big question, Dr. Nolta told me in an interview on July 30, 2012, is this: how effective will MSCs prove in helping the entire striatum, an area of the brain deeply compromised by HD and where the MSCs will be injected?

"The MSCs can secrete huge amounts of BDNF, so that might be effective" in helping to restore the striatum, she said.

Attacking HD's genetic roots

If the MSC BDNF trial proves successful, the UC Davis team could use another up-and-coming tool for combatting HD: RNA interference.

In designing a substance known as a small interference RNA molecule (siRNA), other researchers have already reducedthe amount of harmful huntingtin protein in the brains of test animals. A similar approach, known as antisense, has demonstrated similar results. Both

approaches should enter clinical trials within the next few years, if not sooner.

Still in the early stages of this aspect of their research, Dr. Nolta and her UC Davis HD team have discovered a way to deliver siRNA into cells in a dish using MSCs.

Some researchers are examining ways to implant new neurons or fetal-striatal stem cells into patients' brains to repair the damage caused by HD. However, Dr. Nolta pointed out that those cells could become affected by HD.

The use of siRNA could protect those and other cells from HD. Dr. Nolta has photos and video of the MSC nanotubules transferring siRNA into other cells. Her lab is now testing MSC siRNA in mice.

Controlling the huntingtin gene and protein effectively is the "holy grail" of HD research because it would allow gene-positive, non-symptomatic people like me to take a preventative treatment.

'A super, super clean place'

Although the human brain has MSCs, in HD people those MSCs make the same mutant huntingtin as the other cells in the brain and, indeed, in the rest of the body. Compromised in this manner, the MSCs in HD people's brains cannot make necessary levels of BDNF.

As a result, for the Phase I MSC BDNF trial, the HD team will make batches of MSCs from bone marrow cells provided by a healthy donor and therefore containing normal, non-disease-causing huntingtin.

Federal regulations require GMP for any substance that will be tested in humans. Thus, in the run-up to Phase I, the MSC batches will be made at the UC Davis Institute for Regenerative Cure's GMP facility. It could make enough MSCs for 100 patients, Dr. Nolta said.

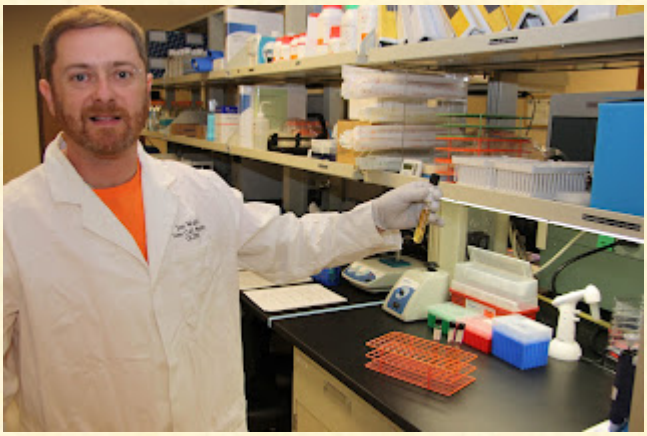
"You need your own facility to get up to this scale," she commented. "How to manufacture these batches of cells is a whole industry in and of itself. It's usually companies that would do this. Sometimes they charge exorbitant fees."

This level of "scale-up" to a clinical trial is "our forte here," Dr. Nolta told me in our recent interview. The National Institutes of Health and insurance companies don't fund these kinds of initiatives, she noted, leading many drug candidates with good potential to "fall into the valley of death."

During my visit to the IRC, she referred to the GMP as a "super, super clean place." It will triple-check the quality of the MSCs.

As explained to me by GMP specialist Bill Gruenloh, normal air contains hundreds of millions of particles per cubic foot. Air handlers and HEPA filters reduce the number of particles in the manufacturing room to only 10,000. Areas under tissue culture hoods have just 100. In addition, the highly specialized GMP employees maintain meticulous records of every article in the facility. A computer constantly monitors the GMP, and the employees double-check readings with hand-held instruments. Thus no micro-organisms are present in critical areas of the GMP.

If a contamination or other problem occurs with a test drug, the GMP records help trace the cause, Gruenloh said.



UC Davis GMP specialist John Walker at work (photo by Gene Veritas)

The GMP also stores stem cells and other items at carefully controlled, very low temperatures. The UC Davis GMP developed the first GMP-grade cell-sorter in the world, Gruenloh added.

In addition, the GMP houses its own quality control lab to check the safety of products and verify that they are free of contaminants and bacteria.

Putting the project in perspective

As Dr. Nolta has pointed out on several occasions, more than 10,000 patients worldwide have already received MSCs infused into the blood stream. In fact, the drug regulatory agencies of Canada and New Zealand have already approved the use of MSCs to be prescribed as a drug to treat certain diseases, although not yet HD. In addition, at least four companies are currently testing MSCs or MSC-like cells in clinical trials for other neurodegenerative conditions.

As always, we need to recall that only 10 percent of clinical trials ever lead to an actual drug. Mathematically speaking,

the odds are stacked against the Nolta-Wheelock project.

Even if the Phase I trial proves a dramatic success, the UC Davis team will need to find ways to fund Phases II and III, which will require larger numbers of participants and thus cost more money. Backed by public bonds, CIRM will run out of money in about four years, unless the agency can attract private investors. At least for now, the state of California's dire fiscal situation makes further public funding unlikely, although one cannot predict the mood of the voters.

With an eye to the future, Dr. Nolta and UC Davis have secured a patent for the MSC siRNA delivery technology in the hopes that a pharmaceutical firm or other private investor might risk supporting further research and testing in exchange for some of the potential profits from a drug. She noted that companies visit the IRC regularly, although none has yet expressed an interest in supporting HD work.

Despite these caveats, I am struck by the apparent simplicity of the UC Davis approach: using human cells as a way to deliver remedies to the brain.

I am also impressed with the UC Davis team's boldness in moving as quickly as possible towards a clinical trial. In fact, some scientists think they're moving too quickly with their siRNA plans, although Dr. Nolta characterized their criticism as a "misunderstanding" of her project, since it is the BDNF trial, not the siRNA, that is moving toward the clinic first. The siRNA studies are only in early rodent testing.

A successful MSC HD trial would extend immense hope to patients suffering from other neurological diseases (such as Alzheimer's and Parkinson's), as well as ischemia, heart disease, and other conditions, Dr. Nolta said. Such hope would likely translate into greater private funding for MSC research.

Hope, realism, and future advocacy

California's HD stem cell advocates – along with fellow HD activists around the world – can feel confident that CIRM is having an important impact on HD research.

We now await the MSC trial results – and with great hope!

However, we should also proceed with patience and realism.

Science takes time.

Furthermore, most scientists think that treating HD successfully will require a *cocktail* of remedies, not just one.

With grassroots support for, and intense interest in, the UC Davis HD program, the HD community is betting heavily that MSCs will provide a way to alleviate the conditions' horrific symptoms.

Judging from the unprecedented excitement about the CIRM grant that I have witnessed in the HD Facebook community in comparison with news about other breakthroughs, I think people perceive stem cells as providing the greatest hope. Indeed, for many Americans, stem cells seem to hold an almost magical appeal, as they once did for the young Jan Nolta at the start of her career. People seem to sense viscerally that they can provide cures and replace lost cells and tissues. Could stem cells represent our new Fountain of Youth?

Naturally, we all want, need, and deserve to celebrate the CIRM award.

I myself have advocated for California stem cell research for more than a decade through HDSA-San Diego. Having lost my mother to HD in 2006 at the age of 68 and tested positive for HD in 1999, I anxiously await treatments. When people told me that potential stem cell breakthroughs lay too far in the future to offer me hope, my resolve to fight only strengthened.

Yet we should also keep in mind that scientists are working just as hard on numerous other, highly important approaches. They don't stir the controversy and publicity that have surrounded stem cells, and many are extremely difficult to understand, but they could very well lead to effective treatments.

In effect, the Nolta-Wheelock project is another "shot on goal" in the search for HD treatments. The CHDI Foundation,Inc., the major private backer of HD drug research, and its collaborators will attempt as many as eight such shots in the next few years. The more shots, the better the chances of finding treatments and a cocktail.

In the meantime, just as Dr. Nolta, the UC Davis team, and scientists around the world work feverishly to liberate us from HD, we in the HD community must continue to strategically advocate for our cause, creatively help change the course of science, and participate in the crucial research studies and clinical trials that provide the key to defeating HD.

* * *

Additional information

Once the UC Davis trial is approved the FDA, details of how to participate will become available at www.clinicaltrials.gov.

For an HD family member's account of the historic CIRM meeting, read Katie Jackson's report at *The Huntington's Post*.

To learn more about Dr. Nolta's research, read an article by Dr. Marsha Miller by clicking here.

For the official CIRM evaluation of the project, please click here.

For in-depth reporting on CIRM's activities, see *California Stem Cell Report*.

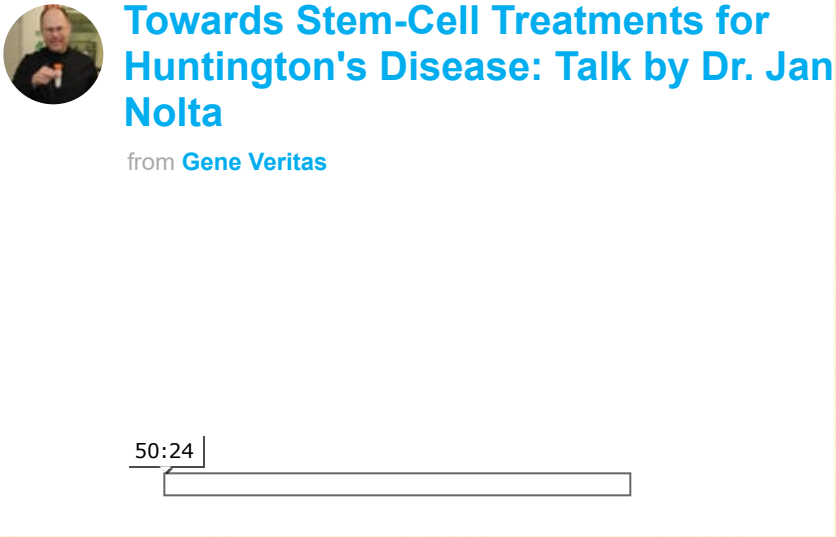
You can also read an impassioned defense of stem cell research by global HD advocate Charles Sabine.

HD scientist Dr. Elena Cattaneo provides an update on the European Union's support for stem cell research.

For an overview of stem cells, see *Stem Cells for Dummies*.

On stem cells and HD, also see www.HDBuzz.net.

To see a presentation by Dr. Nolta on MSCs and HD, watch the video below.



Towards Stem-Cell Treatments for Huntington's Disease: Talk by Dr. Jan Nolta from Gene Veritas on Vimeo.

Posted by Gene Veritas at 9:12 PM

Labels: advocate , antisense , BDNF , brain , CHDI , CIRM , clinical trial , cocktail , drug , gene therapy , huntingtin , Huntington's , mesenchymal stem cell , neurodegenerative , RNA interference , siRNA , stem cell , striatum

1 comment:

stemcelltherapy said...

There is no doubt that medical science has achieved a number of mile stones, especially at the time of emergency but at the same time it is also true that its medication has a number of side effects. So at that moment stem cell therapy can be considered as a perfect solution that to without any medication. Thank you.

stem cell center

1:30 AM, February 19, 2013

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