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Reported December 24, 2014

Stem Cells: A Weapon for Huntington's

Jan Nolta, Ph.D., Professor and Director of the Institute of Regenerative Cures at UC Davis, talks about new developments in delaying the progression of Huntington's disease.

Interview conducted by Ivanhoe Broadcast News in September 2014.

Is using stem cells to treat Huntington's a new field?

Dr. Nolta: It is. The type of stem cells with which we're working, they're adult stem cells from the bone marrow and we've engineered them to make a growth factor that's like a fertilizer for the neurons, it's called brain derived neurotrophic factor and they're like little UPS delivery vehicles that will take these little packages of the fertilizer to the neurons in the brain. It works well in animal models, so we hope that this would help the patients.

What kind of stem cell exactly is it?

Dr. Nolta: We work with mesenchymal stem cells from the bone marrow and these are a type of broad flat cell that support the other cells in the bone marrow. I've been working with these cells for almost 30 years. We've learned over the years that these cells are very good at supporting and nurturing other cell types including neurons. We use those from an adult donor bone marrow and grow them out in our good manufacturing practice facility, expand them to large numbers.

When you're using these, do you have to use your own stem cells or would you use other people's stem cells? Then would you have to take medicine for the rest of your life because you have donor cells?

Dr. Nolta: These stem cells are from a normal donor and they have the amazing property that they shield themselves from the immune system. The recipient of these stem cells would not have to take immune suppressive drugs like you would for a bone marrow transplant or a kidney transplant, or something like that. The cells are very good at shielding themselves from the immune system; it's almost like they're little stealth vehicles to deliver the growth factors to the neurons.

You take stem cells and then you add something to them?

Dr. Nolta: We get the mesenchymal stem cells from a sample, normal donor bone marrow; a donor that's been very well qualified. They can't have any viruses; they can't have any risk of prions, anything like that. We take a little sample of that bone marrow into our good manufacturing practice facility and then we engineer those cells to over express the brain derived neurotrophic factor or BDNF which is like the fertilizer for the neurons that are at risk in Huntington's disease. Huntington's patients have very low levels of this BDNF and we need to get more of that back into the brain and this is one of the good strategies for replacing the levels of that growth factor for the neurons in the brain.

The low levels, what does that cause in a Huntington's patient?

Dr. Nolta: Low levels of BDNF in Huntington's patients are part of why their neurons are dying so rapidly. As their neurons die, patients get movement disorders, they get cognitive and other problems. The brain derived neurotrophic factor actually causes new neurons to be born in the brain and this works very well in animal models. When we put BDNF into Huntington's mouse models, for instance, there are new neurons that are being created in the brain and so we hope that we can replace some of those neurons that have been lost and damaged in Huntington's disease for our patients.

I think that's cool when you can actually do something in the brain where you're replacing – do you replace or repair?

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Dr. Nolta: BDNF is needed for the neurons to talk to each other and to make the synaptic connections and it's also needed for the neuroprogenitors that are located in part of the brain called the SVZ to come forward and differentiate into mature neurons. In Huntington's patients neither of those things are happening; the neurons can't connect with each other properly and the new neurons can't be born.

Does this drug create new ones or and is it kind of like, a detour around the damaged ones? Or do they fix the damaged one?

Dr. Nolta: In the brain there's a little area called the subventricular zone and some people call it the brain marrow, like the bone marrow, because that's where all of the primitive neuro-precursors are living and as they differentiate during development, they will travel back and populate the brain with new neurons. This also happens to a limited degree in adults, and in Huntington's disease it really can't happen because they have such low levels of this brain derived neurotrophic factor. We're replacing the BDNF using our stem cells as the delivery vehicles. We hope that having more of that growth factor or fertilizer in the area of the striatum where it's needed will cause the new neurons to be born from the stem cells within the brain for the adult Huntington's patients. We're also working on other therapies for juvenile Huntington's disease.

Could this be a repair for things like Alzheimer's as well and Parkinson's?

Dr. Nolta: It's interesting, for Parkinson's disease, there's a different cell type; it's needed in a different part of the brain and, in Parkinson's, we don't know that they have deficiencies in brain derived neurotrophic factor. In Huntington's disease, they definitely have very low levels of BDNF and that's because the mutant protein in Huntington's disease acts to stop the brain derived neurotrophic factor from being made.

How does this work? Are you infused once? Is it just an IV?

Dr. Nolta: Our clinical trial will involve putting the stem cells making the growth factor directly into the brain, into the striatum, and it sounds really scary, but it's just a small little burr hole; it would be put on both sides of the brain and the cells really travel very well. The MSC's, the little delivery trucks, we call them the paramedic vehicles as well, they move very well throughout the brain and deliver this factor. We're planning a one-time injection at this point in our planned future trial.

It's just an injection with a large needle?

Dr. Nolta: Yes, under MRI imaging.

Okay.

Dr. Nolta: You would inject.

Was it something that you'd have to redo as the disease progresses?

Dr. Nolta: Yes. Unfortunately, this therapy is not a cure. The Huntington's is still going to progress. We're hoping with this clinical trial to buy the patients a few years of time where their Huntington's wouldn't progress. We, and other labs, are working toward very promising therapeutics for the future where we would shut down the mutant Huntington RNA and protein, and that could, in the future, actually be a cure.

This just stalls the disease and does it kind of stop it in its tracks?

Dr. Nolta: In the mice, it delays progression for about a year which is half of the mouse's lifespan, and we don't know in humans. We're hoping that we would get a significant easing of the symptoms for a significant amount of time. That's why it's a Phase I clinical trial that we're planning.

What type of the symptoms would it help? Are there certain things that it will help and certain things it won't help?

Dr. Nolta: For Huntington's disease, what is most noticed in adult Huntington's disease is the chorea, or the movement disorders, but what the patients would most like help with is the effects on the personality, and happiness. There's a lot of depression and anger that happen and that's the hardest thing to manage. And the brain derived neurotrophic factor and the type of neurons that it stimulates also control emotion and we're hoping that it would make a significant impact on that problem.

Would it have any impact on the shaking and the muscular problems?

Dr. Nolta: We're hoping that it will impact the chorea as well because that happens in the striatum, as the neurons die in Huntington's disease, the chorea is a side effect of that.

Is this just really exciting for you? It feels like any time you can take such a debilitating disease and kind of put the brakes on it for a little bit?

Dr. Nolta: Yeah. My whole life I've worked with this type of stem cell and engineering it to make different growth factors for different tissues, and to be able to work on this for the Huntington's community is, just it really feels like the most important thing in my career ever. I'm so attached to the HD community. There are people I love in this community and we have

a great team working on this. There's just a lot of passion and a lot of excitement for this planned trial.

When does the trial start?

Dr. Nolta: Right now we're in the early part of the clinical trial called PRE-CELL and each patient is their own control because Huntington's progresses at different levels for different people. We're observing future candidates for the HD-CELL trial (when they get the infusion) for 12 to 18 months and we're preparing paperwork for the Food and Drug Administration right now with all of our safety data and our efficacy data to hopefully start at the end of that period.

We're talking to Katy Jackson. Is she going to be part of all this?

Dr. Nolta: Oh great. Yes. Oh she's amazing. She is an important part of it.

Okay. We were talking on the phone and she said this could have a huge impact for her children.

Dr. Nolta: Yes. Juvenile Huntington's disease is a really rapidly progressing form of HD. For juvenile Huntington's disease, we are developing a different strategy where we would stop the ravaging effects of this mutant protein and RNA in its tracks. We're not planning the BDNF therapy for the kids, but we are planning other future therapies for the kids that are just more aggressive, like the disease is more aggressive for them.

Can you share any of that that's going on with the kids?

Dr. Nolta: These are gene silencing techniques, so we're using the same type of stem cells to go in and deliver things that will shut down the mutant gene, but not the normal copy of the gene. And we have that testing in the animal studies as well and we're very committed to the juvenile Huntington's disease community.

What would that mean for these kids? Would they ever even see a symptom if that would work; the gene silencing?

Dr. Nolta: Right now the Food and Drug Administration wants us to treat people who are already symptomatic, so we would hope that we could stop it from progressing further and they would maybe get better in the future, that's our hope and our dream.

How far down the road is helping juvenile Huntington's?

Dr. Nolta: We have an amazing team that's very committed to working on the juvenile Huntington's disease strategies. The research goes as fast as it can. We know that every hour counts for these kids and I don't have a timeline yet, but we will put things forward as soon as it's safe and efficacious to do so.

You can tell that's a subject kind of close to your heart.

Dr. Nolta: It's just heartbreaking. It's bad enough for adults, but some of the JHD kids are six and they're dying from this. It's just not fair.

Any risk right now that you feel with the stem cells?

Dr. Nolta: The stem cells have been shown to be safe in many other disorders. They've been administered systemically in huge doses for other diseases. The fact that we're putting them into the brain, there is a small risk, it's a brain surgery, but this is the same type of surgery that has been used for deep brain stimulation for many, many Parkinson's patients, and has been FDA approved for a decade and it's routine, as routine as brain surgery can get.

Before, stem cells were the hope of the future, and now it feels like the future is here. Because we're actually seeing some of all the research they said, stem cells can do this and do this, but we didn't see it yet.

Dr. Nolta: I would just like to add that the future is now. These therapies are ongoing. We have 10 stem cell clinical trials ongoing right here at UC Davis and we hope that this will be the 11th.

END OF INTERVIEW

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