August 6, 2008

Research News

Scientists Make Stem Cells From ALS Patient

by Joe Palca

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All Things Considered, July 31, 2008 · ALS is a disease of the nervous system. Over time, a special kind of nerve cell called a motor neuron dies, leaving the patient unable to move.

"These nerve cells which die have been impossible to culture in the laboratory, and as a result we have no idea why they die," says Kevin Eggan, a stem cell biologist at the Harvard Stem Cell Institute.

Eggan says one way to get a patient's nerve cells to grow in the lab is to start by making embryonic stem cells from that patient. Embryonic stem cells are pluripotent: They can turn into any cell in the body. So, in theory, you could use them to make nerve cells.

Making embryonic stem cells from adult humans hasn't been possible, however. Not only are there technical hurdles that haven't been overcome, but some people oppose the very idea for moral reasons.

A few years ago, a Japanese scientist showed you could get cells that were pluripotent like embryonic stem cells but didn't involve making an embryo. You could just add a cocktail of four genes to the adult cells you wanted to transform — and voila.

Eggan and his colleagues tried the technique on skin cells taken from an 82-year-old woman with ALS, which is also known as Lou Gehrig's disease.

"We actually infect them with four different viruses, each of which contains a particular embryonic gene," he says. "And then that has this desired effect of transforming those adult skin cells into these pluripotent stem cell lines, which can make all the different cells in the body — including the very specific type of nerve cell which dies in Lou Gehrig's disease."

Eggan presents his work in the latest edition of the journal *Science*. Eggan does not expect the nerve cells he's growing from this patient to be useful for treating her disease, because the genes and viruses used to transform the patient's skin cells are known to cause cancer.

"Until we find some way to circumvent that difficulty, which ... is just a matter of time, we won't be able to transplant these exact cells into patients," he says.

But the cells can be useful since, in theory, they are the same as the ones that die in ALS patients.

"Now we'll literally be able to make a limitless supply of those and ask why that is, and I think invariably that is going to lead to a better understanding of the disease and then, in turn, new drugs for the disease," Eggan says.

There's reason for Eggan's optimism.

Clive Svendsen, who studies ALS at the University of Wisconsin, says it makes sense that if you can study a cell in the lab, you can understand it better. But that hasn't happened yet.

"That is the next stage of the work," he says. "We can generate the neurons. Now we have to find out: Are they any different, and what can we learn about the disease from those motor neurons?"

Svendsen says other research teams are trying to use the same technique the Harvard team used to grow cells specific to patients with other diseases.

"We're going to have a model or a new system in which to study the disease ... and I think you'll see different papers coming out on different disorders, such as Parkinson's and other diseases, in the near future," he says.

But as Svendsen says, then comes the hard part of trying to unravel the nature of these diseases and finding effective ways to treat them.

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