

UM researcher to test stem cell treatment for Alzheimer's

Results from ALS trials spur optimism

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By Ryan Beene And Tom Henderson

Buoyed by early results of stem cell-based trials on patients with Lou Gehrig's disease, Eva Feldman, M.D., co-director of the **A. Alfred Taubman Medical Research Institute** at the **University of Michigan Medical School**, is now taking aim at a far bigger target: Alzheimer's disease.

In late April, Feldman began raising \$1.5 million from private donors to fund animal trials for a stem cell-based treatment of Alzheimer's, a progressive degenerative disease that severely impacts brain function and afflicts more than 5.3 million people in the U.S. It is the seventh-leading cause of death in the nation.

Animal trials are required before Feldman can begin Phase I **U.S. Food and Drug Administration** trials for Alzheimer's on humans. Tests on both safety and efficacy are done first on small rodents and then, if successful, on larger mammals.

Feldman said she hopes to apply for approval in 2013 for human Alzheimer's trials and begin them in 2014.

The investigation into an Alzheimer's treatment piggybacks on current Phase I human trials for patients with Lou Gehrig's disease led by Feldman that are under way at **Emory University Hospital** in Atlanta.

The trials test the safety of injecting neural progenitor cells, essentially stem cells that have developed beyond the embryonic phase and are predisposed to becoming nerve cells, into the spinal cords of patients with Lou Gehrig's disease.

Feldman will continue to serve as principal investigator on that trial — the first FDA-approved trial using stem cells on Lou Gehrig's patients in the U.S. — as she and her team begin work on Alzheimer's trials.

Eighteen Lou Gehrig's patients will be tested in all. The disease, known formally as amyotrophic lateral sclerosis, or ALS, afflicts as many as 30,000 patients in the U.S.

Feldman sped up her timetable for taking on Alzheimer's after seeing promising early results with three Lou Gehrig's patients. The first patient was injected on Jan. 19. The third operation, on April 14, was filmed by **CNN**.

Feldman said she is prohibited from discussing whether patients report such results as increases in strength or sensation. But there have been no ill effects from the three surgeries.

Each patient is injected at five spots on the spinal cord, with about 100,000 cells per injection.

Feldman said she is excited about expanding stem cell trials to Alzheimer's because of the far larger pool of would-be patients.

“Alzheimer's is going to be easier to do than ALS,” said Feldman.

She said that the brain can be injected with far more stem cells than the spinal cord, promising greater and faster benefits, and she said the surgery is far less invasive. Instead of needing to remove bone from the back, a tiny hole is cut into the skull in a relatively safe, easy procedure.

The transition from Lou Gehrig's to Alzheimer's disease is a natural one because the treatment potentially addresses the same problem. The neural progenitor stem cells work by surrounding

specific large nerve cells that are sick and halting further degeneration caused by the disease, Feldman said.

“In the spinal cord, these nerve cells produce the nerve tissue fibers that extend through the muscles of our body, and in the brain, the same type of nerve cell facilitates thinking processes,” Feldman said.

“The kind of stem cells we're using have a particular proclivity to rescue cholinergic neurons, and it's cholinergic neurons that degenerate and become diseased in Lou Gehrig's disease and Alzheimer's disease.”

The surgeon in the current trials is Dr. Nicholas Boulis, an associate professor at Emory University who was formerly a fellow in Feldman's research lab at UM.

Boulis specializes in movement disorders, such as Parkinson's and Huntington's diseases, and performs about 300 operations a year. He also heads a gene-therapy research lab and is involved in a project that aims to use gene therapy to treat Alzheimer's.

Boulis said he hopes, if the FDA approves human tests, to do Feldman's Phase I Alzheimer's operations, too.

“If Eva thinks we can make progress, I'm her man,” he said.

The Phase I Lou Gehrig's disease trials are scheduled to finish by the end of June 2011. If they go as hoped, Phase II trials, which assess efficacy, can begin as early as January 2012. Feldman said Phase II trials could add the UM hospital as a test site in addition to Emory.

Investigating a treatment for Alzheimer's using stem cells is an “interesting approach” and a logical next step to investigate, said Dr. Ken Maiese, professor in the departments of neurology and anatomy and cell biology at **Wayne State University Medical School**.

“There's really no good treatment for Alzheimer's, although there are many trials going on” for drugs that deal with chemicals in the brain related to Alzheimer's, Maiese said.

But those treat the symptoms, not the underlying issue of rapid brain cell degeneration that is a hallmark of Alzheimer's.

Maiese cautioned that the science behind a stem cell treatment still has a long way to go, as in any treatment. Going from animal to human trials involves many unknowns.

Feldman said she recently took on a new, young ALS patient, to whom she could, for the first time in her 20 years of treating patients at UM, offer some encouraging words about future treatments.

“For 20 years, there has been little hope I could offer patients. Now there is truly tangible hope. We are truly beginning to try a therapy that can allow us to help halt the progress of this dangerous disease,” she said.

“Patients ask me “what will the future hold?” I told my new patient, things are extremely hopeful now. The future is very bright. And not just with ALS or Alzheimer's, but with Parkinson's and Huntington's, too.”

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