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ALS stem cell trial begins at U-M Health System with first two patients receiving injections

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Two patients with amyotrophic lateral sclerosis (ALS) have received stem cell injections to their spinal cords at the University of Michigan Health System – the first two to receive the experimental injections in Michigan as part of a national clinical trial.

Both research volunteers have returned home and will receive follow-up monitoring and testing to help U-M researchers assess the safety and any potential effect of the injections.

Additional patients with the condition, also known as Lou Gehrig's disease, are being evaluated for possible participation in the trial at U-M and Emory University.

The Phase II trial is approved by the U.S. Food and Drug Administration and funded by Neuralstem, Inc., the Maryland-based company whose stem-cell product the trial is testing. It seeks to study any effect that injected stem cells might have on motor neurons – muscle-controlling nerve cells that die in ALS patients, eventually robbing them of the ability to walk, speak and breathe.

Eva L. Feldman, M.D., Ph.D., the Russell N. DeJong professor of neurology at the U-M Medical School and director of the A. Alfred Taubman Medical Research Institute, is the principal investigator for the trial. Feldman serves as an unpaid consultant to the company, and has led

the analysis of results from the Phase I trial which concluded in 2012.

In data presented earlier this year, spinal cord injections of up to 100,000 cells were delivered safely and tolerated well in a Phase I trial conducted at Emory. The researchers reported possible signs that in one subgroup of participants, ALS progression may have been interrupted.

"We're going to be permitted to give more injections and more stem cells, in Phase 2," said Feldman. "We're very excited that we have been able to bring this important work to the University of Michigan."

Parag Patil, M.D., Ph.D., a U-M neurosurgeon and biomedical engineer, performed both operations on the U-M trial participants. In each case, the patient's spinal column was unroofed and the spinal cord exposed to receive the cells. The cells are introduced via a custom-designed delivery device that is affixed to the subject's spinal bones so that it moves with the patient's breathing throughout the process.

Patil, an assistant professor in U-M's departments of Neurosurgery, Neurology, Biomedical Engineering and Anesthesiology, and a Young Friends of the Taubman Institute Emerging Scholar, also serves as a paid engineering consultant to Neuralstem to further develop the cell-delivery device.

A third participant in the Phase II trial received the same surgery in September at Emory University in Atlanta, the other site for the trial.

This Phase II dose escalation trial is designed to treat up to 15 ambulatory patients in five different dosing cohorts, under an accelerated dosing and treatment schedule.

The first 12 patients, divided into four cohorts, will receive injections only in the cervical region of the spinal cord, where breathing function is

controlled. The first cohort of three patients received 10 cervical region injections of 200,000 cells per injection. The trial will now progress to a maximum of 20 cervical injections of up to 400,000 cells per injection.

The last three Phase II patients will receive injections in both the cervical and the lumbar spinal regions. These patients will receive 20 injections of 400,000 cells each in the lumbar region in addition to the 20 injections they will already have received in their cervical region.

The trial also accelerates the treatment schedule, and is designed to progress at the rate of one cohort per month with one month observations periods between cohorts. Researchers expect all of the patients could be treated by the end of the second quarter in 2014.

More information: umhealth.me/UM-ALS

Provided by University of Michigan Health System

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