Embryonic stem cell therapy for paralysis given to first patient in western United States

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The Stanford University School of Medicine and Santa Clara Valley Medical Center have enrolled the fourth participant in the nation's first trial of cells derived from human embryonic stem cells. The phase-1, FDA-approved trial is meant to test the safety of the cells in up to 10 people with recent spinal cord injuries at seven trial sites across the United States.

The most recent patient was treated Sept. 17 at the Rehabilitation Trauma Center at SCVMC with cells prepared for injection at Stanford. Stanford neurosurgeon Gary Steinberg, MD, PhD, implanted the cells. Three other patients have previously received the surgically delivered cells: two at the Shepherd Center in Atlanta beginning in October of last year, and one at Northwestern Memorial Hospital and the Rehabilitation Institute of Chicago in May 2011. The Stanford/SCVMC patient is the first person to receive the therapy west of the Mississippi.

"We are extremely excited to participate in this landmark clinical trial," said Steinberg, who is the Bernard and Ronni Lacroute-William Randolph Hearst Professor in Neurosurgery and Neurosciences at Stanford and the principal investigator of the Stanford/SCVMC portion of the trial. "It signifies a major advance in translating an innovative research discovery into clinical therapy. I believe it is critically important to encourage and take part in stem cell trials like this, which represent a new era in the effort to restore function for patients with stroke, brain injury, Parkinson's disease and other devastating neurologic disorders."

Those sentiments were echoed by Stephen McKenna, MD, chief of the rehabilitation center at SCVMC. “It has been an extraordinarily collaborative process at every step, from developing the screening process and identifying possible patients to evaluating these patients for surgery,” McKenna said. “Although it’s been an intensive commitment of resources, we understand the importance of advancing new therapies for patients.”

The trial is being run by Geron Corp. of Menlo Park, Calif., which developed and manufactures the cells being tested. In May, Geron received a $25 million grant from the California Institute for Regenerative Medicine to continue and extend the trial to include a greater proportion of spinal cord injuries.

“When the people of California voted in favor of Proposition 71, they did so with the hope of seeing stem-cell-based therapies for chronic disease and injuries. This first California patient to participate in Geron’s landmark spinal cord injury trial is a major step toward fulfilling that hope,” said Jonathan Thomas, chair of the CIRM governing board. “We are proud to be providing funding for this first safety trial, which is a critical step toward making safe and effective stem-cell-based therapies available to patients.”

Researchers at Geron collaborated with Hans Keirstead, PhD, and his laboratory team at UC-Irvine to develop a way to coax human embryonic stem cells to become a mixture of cells that include oligodendrocyte precursors. Oligodendrocytes are cells in the brain and the central nervous system that wrap nerve cells with an insulating material called myelin. This myelin sheath is necessary for the transmission of the electric signals along the spinal cord that trigger muscles to move, and relay our sense of touch and temperature. Damage to this sheath caused by trauma is a common cause of paralysis.

To be eligible for the trial, patients must have recent (within 14 days of injury) non-penetrating damage to a specific region of their thoracic spine — an area roughly from the top of the shoulder blades to the bottom of the rib cage. The damage must cause complete paraplegia, meaning that they have normal sensation or movement to the level of the hands, but not from the trunk to the toes.

During the procedure, Steinberg applied about 2 million of the special cells, called GRNOPC1, directly into the injured area of the patient's spinal cord.

"We are quite pleased that the surgery was completed successfully and the patient is doing well," said Steinberg.

Following the surgery at SCVMC, the patient entered an intensive inpatient rehabilitation program under the supervision of McKenna and James Crew, MD, who are specialists in spinal cord injury medicine. Researchers will now monitor the patient for any adverse events to confirm that the cells are safe for use in humans.

"In the future, cellular therapies such as those used today will open new hopes for a cure to catastrophic neurological injuries," said McKenna. "Our institution is the first rehabilitation hospital in the western United States delivering human embryonic stem cell treatments for spinal cord injury. Kaiser Permanente demonstrated a strong commitment to research by transferring the patient to Valley Medical Center where this trial is being conducted."

In June, Geron reported preliminary results of the trial on the first two patients at two meetings: the 2011 International Conference on Spinal Cord Medicine and Rehabilitation and the 2011 Spine Symposium. The results so far show no significant adverse effects experienced by either patient. If this phase-1 trial of 10 patients shows that the treatment is

safe, future trials will be designed to determine whether the cells are able to improve participants' clinical symptoms.

Provided by Stanford University Medical Center