US treats first patient with human embryonic stem cells (Update 2)

October 11 2010, by Karin Zeitvogel

US doctors have begun the first tests of human embryonic stem cells in patients, treating a man with spinal cord injuries in a landmark trial of the controversial process, the Geron Corporation said Monday.

The patient began the pioneering treatment Friday with an injection of the biotech company's human embryonic stem cells, as part of a clinical trial that aims to test safety and efficacy toward regaining sensation and movement.

The treatment took place at the Shepherd Center in Atlanta, Georgia, a spokeswoman for the hospital told AFP, declining to give further details due to patient privacy concerns.

The Phase I trial is expected to involve around 10 patients. Participants in the human trials must be severely injured and start treatment with Geron's product, GRNOPC1, seven to 14 days after sustaining their injury.

Patients will be given a single injection of two million of Geron's GRNOPC1 cells in the trial.

Those taking part will be followed up for one year to monitor safety and also to see if they have regained any sensory function or movement in their lower extremities.

If the initial group of subjects shows no negative side-effects, Geron

plans to seek FDA approval to extend the study to increase the dose of GRNOPC1 and to include patients with "as broad a range of severe spinal cord-injured patients as medically appropriate."

The ultimate goal for GRNOPC1 is to inject it directly into the spinal cord lesions of injured humans where it would, Geron hopes, prompt damaged nerve cells to regrow, enabling patients to eventually recover feeling and movement.

Geron began working with human embryonic stem cells in 1999.

Back then, "many predicted that it would be a number of decades before a cell therapy would be approved for human clinical trials," Geron's president and chief executive Thomas Okarma said in a statement.

Okarma described Monday's start of the clinical trial as "a milestone for the field of human embryonic stem cell-based therapies."

GRNOPC1 is made up of cells containing precursors to oligodendrocytes -- multi-tasking cells that occur in the nervous system.

Oligodendrocytes are lost in spinal cord injury, resulting in myelin and neuronal loss which cause paralysis in many patients.

Preclinical studies of GRNOPC1 found that when it was injected into the injury site of animals with spinal cord injuries, it migrated throughout the lesion site and matured into oligodendrocytes.

Those oligodendrocytes then re-lined axons with myelin, the insulating layers of cell membrane that wrap around the axons of neurons to enable them to conduct electrical impulses.

The process produced biologicals that enhance the survival and function

of neurons, resulting in significantly improved locomotion in the treated animals.

In the animal trials, GRNOPC1 was injected seven days after the injury was sustained.

Every year, some 12,000 people in the United States sustain spinal cord injuries, usually in automobile accidents or from falls, gunshot wounds and sports.

Geron got clearance in January 2009 from the Food and Drug Administration to conduct human trials of GRNOPC1.

Around six weeks later, President Barack Obama reversed a ban on federal funding for research on human embryonic stem cells, which had been imposed by his predecessor at the White House, George W. Bush.

But the clinical trials of GRNOPC1 remained on hold for more than a year while the US courts wrangled about whether lifting the ban on embryonic stem cell research was legal.

Backers of the research believe the field holds huge potential for treating serious diseases including cancer and Alzheimer's, and even for reversing paralysis.

Opponents argue that living embryos are destroyed in order to obtain the potentially life-saving embryonic stem cells.

Legislation passed by Congress in 1996 bans federal funding for research in which human embryos are either destroyed or discarded.

In lifting the ban on embryonic stem cell research, the Obama administration argued the research does not require disposal or

destruction of the embryos, which were created for in-vitro fertilization treatments but never used.

Last month, a US appeals court ruled that the federal funding can continue, dissolving a lower court's ban.

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