US clears embryonic stem cell trial for blindness (Update 3)

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US biotech company Advanced Cell Technology said Monday it was cleared by government regulators to start its second trial using human embryonic stem cells to treat blindness, this time in older people.

The trial will examine the therapy's ability to safely treat people with a condition known as dry age-related macular degeneration, the most common form of irreversible vision loss in people over age 60.

There is currently no cure for the disease, which affects around 10-15 million Americans and another 10 million people in Europe, the company said.

Embryonic stem cell research has been a controversial field ever since the first such stem cells were isolated more than 12 years ago. Critics oppose the research because it involves the destruction of human embryos.

However scientists say the cells offer great promise in treating Parkinson's disease, diabetes and a variety of other illnesses.

The Food and Drug Administration cleared the Massachusetts-based company in November to begin a similar trial on patients with a less common form of juvenile vision loss, known as Stargardt's disease.

"ACT is now the first company to receive FDA clearance for two hESC (human embryonic stem cell) trials, and is now a true translational leader

in the field of regenerative medicine," said chief executive Gary Rabin.

"It marks a major step forward, not just within the stem cell sector, but, potentially for modern healthcare techniques."

The company hopes to begin the US clinical trials in the coming months, and intends to seek approval for similar trials in Europe. The US and European market for such a treatment amounts to 25 to 30 billion dollars, it said.

"We are moving ahead aggressively to seek regulatory clearance from the European Medicines Agency to conduct clinical trials in Europe," said Edmund Mickunas, ACT's Vice President of Regulatory Affairs.

ACT's announcement marks the third such trial of embryonic stem cells in human patients, after US company Geron broke new ground in October last year with the first-ever attempt to use the therapy on a human patient with spinal cord injury.

Like the other trials involving human patients, the first step in ACT's Phase I and II trials is to assess whether the therapy is safe before moving on to testing how well it works.

Bob Lanza, chief scientist at ACT, said that trials using the technique on animals have shown no evidence of complication or tumors.

"In a rat model of macular degeneration, we have seen a remarkable improvement in visual performance over untreated animals, without any adverse effects," said Lanza.

Twelve patients will enroll in the study at various US sites including the University of California, Los Angeles (UCLA) and Stanford University.

The therapy uses retinal pigment epithelial (RPE) cells derived from human embryonic stem cells to replace RPE cells that have broken down in patients with the disease.

Dry age-related macular degeneration, the type that occurs in 90 percent of cases, causes a deterioration in central vision when RPE cells in the patient's macula, in the center of the retina, lose their ability to function.

Patients often experience blurring in the center of their field of vision, while peripheral view remains intact.

Dry AMD gets progressively worse, while the other type, known as wet AMD, does not advance in stages. Wet AMD is caused by abnormal blood vessels which leak fluid behind the macula, causing a quicker loss of central vision.

"As the population ages, the incidence of AMD is expected to double over the next 20 years, further exacerbating this unmet medical need," Lanza said.

Former president George W. Bush outlawed federal funding for embryonic stem cell research on the basis that it destroyed human life, a ban that President Barack Obama reversed shortly after taking office in 2009.

Private companies ACT and Geron have been able to avoid much of the controversy associated with the technology by securing their own funding for the early trials, as well as by meeting stringent government regulations.

"We really feel like we have looked at every single possible angle," said Rabin, who said he is "optimistic" that the trials will be able to expand to France and Britain, where no embryonic stem cell technology has yet

been tried on humans.

Rabin estimated the company has spent around 30 million dollars in preparation for the trials and will spent between four and five million on each Phase I/II trial for Stargardt's and AMD.

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