Europe's first human embryonic stem cell trial approved

September 22 2011, by Kerry Sheridan

A US biotech company said Thursday it will soon begin the first-ever European trials using human embryonic stem cells in an experimental treatment for people with a form of juvenile blindness.

The Massachusetts-based Advanced Cell Technology said the trials will involve 12 patients with Stargardt's disease and will be based at Moorfields Eye Hospital in London, with more European sites planned for the future.

"This is the first time an embryonic stem cell trial has ever been approved anywhere else in the world," said Bob Lanza, chief scientific officer at ACT and a longtime researcher in human embryonic stem cells .

The clearance to begin the European trials came from the UK Medicines and Healthcare products Regulatory Agency and the Gene Therapy Advisory Committee, ACT said.

The same company became the first to launch a US trial of embryonic stem cells to treat Stargardt's disease in November 2010, followed in January by a second trial of the method in patients with dry age-related macular degeneration.

So far, only two US patients have been treated as part of those early trials which are mainly aimed at seeing if the treatment is safe before moving on to measure if or how well it works.

"We're very pleased with the results so far. We're in the process of scheduling the next two patients for each of the two (US) trials," Lanza told AFP.

The use of human embryonic stem cells, which can become any cell in the body, has been touted by researchers as wielding great regenerative potential against a host of disorders, from spinal cord injuries to Parkinson's disease, blindness and diabetes.

However, the technology has raised objections by conservative and religious opponents who say it should be banned because the cells' extraction involves the destruction of a human embryo.

Former president George W. Bush had blocked government funding for human embryonic stem cell research on new cell lines, citing religious grounds, a ban which President Barack Obama lifted in 2009.

A lengthy legal battle ensued, and in July a US federal judge finally dismissed a lawsuit that had temporarily blocked government funding for the research.

The move was hailed by the National Institutes of Health, which allocated about $40 million to human embryonic stem cell research in 2010 and has set aside $125 million this year -- a tiny fraction of its $31 billion budget.

As private companies, ACT and Geron, which last year began a US trial using the cells to treat paralysis, have been able to avoid much of the controversy by securing their own funding for the early trials, as well as by meeting stringent government regulations.

Another major concern about the research is the possibility that the stem cells could form into tumors, but ACT said it has seen no evidence of

that in its trials so far.

ACT's method involves using human embryonic stem cell-derived retinal pigment epithelium (RPE) cells, or the pigmented layer of the retina.

The cells are injected into the eye of a patient whose RPE cells have broken down.

Patients with Stargardt's disease, formally known as Stargardt's Macular dystrophy, often experience blurry vision, difficulty seeing in low-light and eventually most lose their ability to see at all.

The disease can be inherited by a child when two parents carry the gene mutation that causes it. There is no cure.

The treatment process being tested by ACT worked in animals by creating an abundance of new RPE cells.

Tests on rats have shown 100 percent improvement in visual performance and "near-normal function" was also achieved in mice, both without negative side effects, ACT said.

Between 80,000 and 100,000 people in the United States and Europe are estimated to suffer from Stargardt's, which is one of the most common forms of juvenile blindness in the world and can take root as early as age six.

If the treatment is proven to work in humans, ACT believes it could extend to a much wider market.

That could include other degenerative diseases of the retina such as macular degeneration, which affects as many as 30 million people in the US and Europe and "represents a $25-30 billion worldwide market that

has yet to be effectively addressed," the company said in a statement.

(c) 2011 AFP