Study: Stem cells may aid vision in blind people

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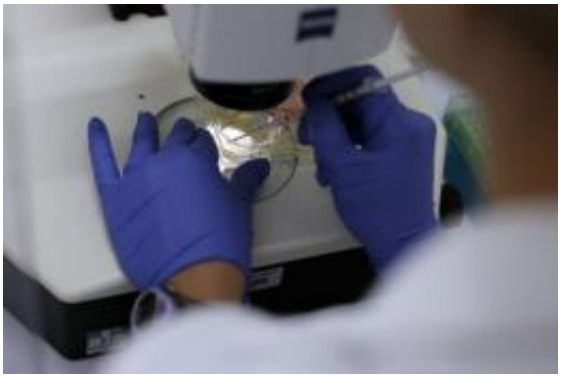

Assistant Professor of Genetics and Developmental Biology Stormy Chamberlain works on stem cells at the University of Connecticut's Stem Cell Institute in 2010 in Farmington, Connecticut. Embryonic stem cells are extraordinarily versatile cells, found in early-stage embryos, that can differentiate into any tissue of the body.

The first use of embryonic stem cells in humans eased a degenerative form of blindness in two volunteers and showed no signs of any adverse effects, according to a study published by The Lancet on Monday.

Publication in the peer-reviewed journal marks an important step for embryonic stem cells, which were hailed as a miracle cure after they were discovered in 1998 but then ran into technical and political hurdles.

The results of the cautious first-stage test, designed to evaluate whether

the treatment is safe, had been previously announced by Massachusetts biotech firm Advanced Cell Technology (ACT) Inc.

The positive outcome in the United States opened the way to the first trials in Europe, which began on Monday.

Embryonic stem cells are extraordinarily versatile cells, found in early-stage embryos, that can differentiate into any tissue of the body.

Scientists have been hoping to turn them into replacement for tissue lost through disease or lost in accidents or war.

The quest to use embryonic stem cells has been arduous.

One problem is biological: that donated stem cells, provoking an immune response, can be rejected by the body or cause cancer. The other is ethical, with moral conservatives contending that an embryo is a human life.

Addressing the biological question, ACT used the stem cells at a so-called "immunoprivileged" site, the eye, where there is not a strong immune response because of a shield known as the blood-ocular barrier.

Around 50,000 embryonic stem cells that had diversified into replacement cells for the pigmented layer of the retina were transplanted into two legally-blind volunteers.

One, a woman in her 70s, had a condition called dry age-related macular degeneration, the leading cause of blindness in the developed world; the other was a woman in her fifties who had Stargardt's macular dystrophy, the commonest form of vision loss among young people.

For the next six weeks, the patients received treatment to prevent their

immune systems from attacking the implanted cells, but this was gradually scaled back.

In the first four months, no signs of cancer, rejection or other safety concerns emerged and both patients recovered a little vision, although this was not the point of the test.

At the outset, the older patient was able to read 21 letters on a standard chart of visual acuity. This rose to 33 letters after two weeks before settling at a stable ability to read 28 letters, the study said.

The woman with Stargardt's disease, a former graphic artist, at first could only see hand movements, but this improved after the transplant to being able to see single fingers and to reading five letters of the alphabet.

"However, that doesn't really capture the difference it has made in their life," Bob Lanza, ACT's chief scientific officer, said in an email to AFP.

"The Stargardt's patient reports that she can see more colour and has better contract and dark adaptation out of the operated eye. In fact, she started using her computer and could even read her watch... (and) says she can even thread a needle now."

Lanza noted that the improvements occurred in patients who were already at a very advanced state of the disease, so the trials were encouraging for patients at an earlier stage of degeneration.

Clinical trials of novel drugs or treatments typically undergo a three-phase process, enrolling a progressively larger number of patients, to make sure they are firstly safe and, secondly, effective.

The Lancet had been scheduled to publish the study on Thursday but released it on Monday as ACT launched its first European trials of the

retinal treatment.

Twelve patients with Stargardt's have been cleared by British medical authorities to undergo transplant, with progressively higher doses of cells, at the Moorfields Eye Hospital in London.

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