Once they were blind, now they see. Patients treated with cells from human embryo

Controversial medical breakthrough restores vision – now doctors hope to repeat the success

Steve Connor

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Two blind people have shown signs of being able to see again – despite having incurable eye disease – following a revolutionary operation involving the transplant of stem cells derived from a human embryo.

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A third patient, a Yorkshire man who volunteered to take part in a similar trial in Britain, had a similar transplant operation last week involving the injection of embryonic stem cells into the damaged retina at the back of the eye.

The three people are the first wave of patients to receive controversial transplants of embryonic cells as part of an ambitious attempt to treat a range of incurable diseases with stem cells that have the power to develop into any of the dozens of specialised tissues of the body.

The two American patients, who each received transplants in just one of their eyes last year, have not shown any signs of serious side effects, such as tissue rejection or the development of tumours, according to a study to be published later this week in The Lancet.

One of the patients, a woman in her 50s, suffers from Stargardt's macular dystrophy, a progressive disease of the central retina that usually strikes between the ages of 10 and 20. The other, a woman in her 70s, has age-related macular degeneration, the leading cause of blindness in the developed world.

Although both patients have exceptionally poor vision and are legally registered as blind, their sight in the treated eye seems to have improved slightly following the transplants, even though their disease is at an advanced stage and was not expected to recover.

The Stargardt's patient went from only being able to see hand movements to being able to see the movements of fingers, while the age-related patient went from being able to see 21 letters on a reading chart to seeing 28 letters.

"Despite the progressive nature of these conditions, the vision of both patients appears to have improved after transplantation of the cells, even at the lowest dosage," said Robert Lanza, chief scientific officer of Advanced Cell Technology, the Massachusetts company that supplied the cells.

"This is particularly important, since the ultimate goal of this therapy will be to treat patients earlier in the course of the disease where more significant results might potentially be expected," Dr Lanza said.

In a separate clinical trial being conducted in Britain, a 34-year-old Yorkshire man suffering from Stargardt's disease underwent an embryonic stem cell transplant in his right eye last Friday at Moorfields Eye Hospital in London.

Professor Douglas Bainbridge, a consultant surgeon at Moorfields, said the operation was deliberately carried out on the patient's worst eye in order to minimise the risk to his overall vision. There were no adverse reactions and the patient was allowed to travel back to his home in Wakefield yesterday.

"There were no complications and the patient tolerated the surgical procedure well. We will be regularly monitoring the patient to follow the safety and tolerability of these transplanted cells," Professor Bainbridge said.

"While this is still primarily a safety trial, we will also have the opportunity to assess any changes in visual function in the treated eye and look for signs that the injected [cells] engrafted in the retina," he said.

Macular degeneration involves the progressive loss of cells in the retinal epithelium, the tissue layer that supports and protects the light-sensitive cells at the back of the eye. Patients with macular degeneration lose their central vision, which is important for reading and recognising faces.

"It is hoped that cell transplants might play a role in protecting people from sight loss in the future. This is a very early, small step in the development of a new, effective intervention," Professor Bainbridge said.

"This is a safety trial so we are deliberately going for patients with advanced sight impairment to limit the possible damage from the stem-cell transplants. In future we'll be looking to recruit less advanced patients," he said.

Up to 12 patients with Stargardt's disease will be recruited into the phase one safety trial in Britain, which is being run from Moorfields and Aberdeen. Different doses of stem cells will be compared for safety in preparation for a phase two clinical trial designed to assess whether the therapy can significantly restore vision.

The only other phase one clinical trial of human embryonic stem cells was designed to test their safety on patients with spinal cord injury. However, the trial was abandoned last November when the American biotechnology firm Geron announced it was pulling out of the entire field over financial concerns.

http://www.independent.co.uk/news/science/once-they-were-blind-now-they-see-patients-treated-with-cells-from-human-embryo-6293706.html#