

People facing blindness may pioneer stem-cell therapy

Clinical trials are expected to start in spring

Mark Henderson Science Editor

Patients with a rare and incurable eye disease that causes blindness early in adulthood are expected to become the first people to benefit from an embryonic stem-cell therapy.

Clinical trials of the treatment for Stargardt's disease are expected to begin next spring, after an American company applied yesterday for regulatory permission to proceed. If the research is cleared by the US Food and Drug Administration (FDA), the 12 patients with Stargardt's disease included in the trial could become the first in the world to receive any kind of treatment based on embryonic stem cells.

In January the FDA approved a trial of an embryonic stem-cell therapy for spinal paralysis, but there have been problems, and no patients are expected to be treated before late next year.

Robert Lanza, of Advanced Cell Technology, which developed the therapy, described the application as an important advance. "After years of research and political debate, we're finally on the verge of showing the potential clinical value of embryonic stem cells," Dr Lanza said. "Our research clearly



Put to the test: human stem cells

shows that stem cell-derived retinal cells can rescue visual function in animals that otherwise would have gone blind. We are hopeful that the cells will be similarly efficacious in patients."

The application to the FDA could assist research by a British team led by Pete Coffey, of University College London, that aims to use embryonic stem cells to treat age-related macular degeneration, the commonest cause of blindness. Professor Coffey wants to start clinical trials early in 2011.

Embryonic stem cells are master cells found in embryos that can form any of the specialised tissue types in the adult human body. They have great potential as a source of replacement tissue for treating disease or injury, but their use is opposed by some religious groups because they can be created only by destroying embryos.

Advanced cell technology has used a line of embryonic stem cells grown from an embryo to create retinal pigment epithelial cells, a type of eye tissue that malfunctions in Stargardt's disease and age-related macular degeneration. In studies of rats with Stargardt's, implanting these cells led to a substantial improvement in eyesight. The animals showed no adverse effects.

Stargardt's is among the commonest causes of blindness in the young. The condition was diagnosed in Marla Runyan, the American Olympic runner, when she was 9.

Dr Lanza said that the trial would involve 12 patients at three centres in the US, and is designed first to assess safety and tolerability. If this is successful, a larger study to examine effectiveness will follow, and if all goes well a treatment could be approved for wider use within three to five years.

