Embryonic stem cell therapy closer to human trials

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Embryonic stem cell therapy got a step closer to the clinic Thursday after US researchers said they filed a request for government approval of human trials.

The trials would involve 12 patients losing their sight to a currently untreatable disease called Stargardt, which is one of the most common forms of juvenile blindness.

The treatment would consist of a single injection of retinal cells derived from embryonic stem cells.

Previous studies in rats and mice have found that the treatment prevented further vision loss without adverse side effects, said Robert Lanza, the chief scientific officer at Advanced Cell Technology.

It works by replacing lost retinal cells -- called retinal pigment epithelium (RPE) -- which maintain the photoreceptors needed for vision.

"If you start to lose these RPE, the environment for the photoreceptors degenerates," Lanza said in a telephone interview.

"By putting these RPEs back in you can prevent the loss of these photoreceptors and prevent blindness."

The trials could begin as early as the beginning of next year if the Food

and Drug Administration grants approval, Lanza said.

This is just the second proposal for a clinical trial of embryonic stem cell therapies that has been submitted for approval, he added.

The other project, which would address spinal cord injuries, has been placed on hold and is not expected to begin before the third quarter of 2010, according to a recent press release from California-based Geron.

"After years of research and political debate, we're finally on the verge of showing the potential clinical value of embryonic stem cells," Lanza said.

"The field desperately needs a big clinical success."

Embryonic stem cell research is controversial because human embryos are destroyed in order to obtain the primitive cells capable of developing into almost every tissue of the body.

But it also holds great promise for treating cancer, diabetes, Alzheimer's and other diseases and even growing transplantable organs and tissues.

Researchers have found a way to sidestep both the controversy and the difficulty of working with embryonic stem cells by reprogramming ordinary skin cells into stem cells.

But this method is still so new that it could take years to replicate the experiments already done with embryonic stem cells and build up similar banks of tested cell lines, Lanza said.

"That is not a substitute certainly in the next several years for embryonic stem cell therapy," he said.

The Massachusetts-based company will be seeking approval for a human trial using similar methods to treat age-related macular degeneration, Lanza added.

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