

# Stem cell therapy shows promise for rescuing deteriorating vision

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For the millions of Americans whose vision is slowly ebbing due to degenerative diseases of the eye, the lowly neural progenitor cell may be riding to the rescue.

In a study in rats, neural progenitor cells derived from human fetal stem cells have been shown to protect the vision of animals with degenerative eye disease similar to the kinds of diseases that afflict humans. The new study appears today (March 28) in the journal *Public Library of Science (PLoS) One*.

The lead author of the study, University of Wisconsin-Madison researcher David Gamm, says the cells - formative brain cells that arise in early development - show "some of the best rescue, functionally and anatomically" of any such work to date. In animals whose vision would typically be lost to degenerative retinal disease, the cells were shown to protect vision and the cells in the eye that underpin sight.

The new findings are important because they suggest there may be novel ways to preserve vision in the context of degenerative diseases for which there are now no effective treatments. Macular degeneration, an age-related affliction that gradually destroys central vision, is a scourge of old age, robbing people of the ability to read, recognize faces and live independently.

The finding that the brain cells protected the cells in the eye was a surprise, according to Raymond D. Lund, an author of the new study and an eye disease expert at the University of Utah and the Oregon Health and Sciences University. The neural progenitor cells, which arise from stem cells and further differentiate into different types of cells found in the central nervous system, were being tested for their ability to deliver another agent, a growth factor that has been shown to be effective in treating some types of degenerative disease.

What was surprising, say Gamm and Lund, was that the cells alone demonstrated a remarkable ability to rescue vision.

"On their own, they were able to support retinal cells and keep them alive," says Lund, who has conducted pioneering studies of cell therapy for eye disease. "We didn't expect that at all. We've used a number of different cell types from different sources and these have given us the best results we've ever got."

How the cells act to preserve the deteriorating eye cells remains unknown, says Gamm. Like all cells, neural progenitor cells do many things and secrete many different types of chemicals that may influence the cells around them.

"The idea was to test the cells as a continuous delivery system" to shuttle an agent known as glial cell line-derived neurotrophic factor or GDNF, Lund explains. "It's not a sensible thing to inject the eyes many times over years. The idea was to use the cells as a continuous delivery system, but we found they work quite well on their own."

Lund has experimented with other cell types as therapies for preserving vision. The neural progenitor cells, a cell model developed by Wisconsin stem cell researcher Clive Svendsen, have been used experimentally to deliver the same growth factor in models of Parkinson's disease and Lou Gehrig's disease. Svendsen is also an author of the new PLoS One report.

"It seems that the cells in and of themselves are quite neuroprotective," says Gamm. "They don't become retinal cells. They maintain their own identity, but they migrate within the outer and inner retina" where they seem to confer some protection to the light-sensing cells that typically die in the course of degenerative eye disease.

For researchers, the work is intriguing because the

progenitor cells come from the brain itself, and not from the part of the nervous system devoted to vision.

"This cell type isn't derived from the retina. It is derived from the brain," says Gamm. "But we're not asking it to become a retina. They survive in the environment of the eye and don't disrupt the local architecture. They seem to live in a symbiotic relationship" with retinal cells.

Gamm and Lund emphasize that the new work is preliminary, and that much remains to be done before the cells can be tested in humans: "The first thing is to show that something works, which we have done," says Lund. "Now we need to find out why, but this is a good jumping off point. "

Source: University of Wisconsin-Madison

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