

Researchers find way to prevent blindness in research model for retinitis pigmentosa

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Researchers at the University of Oklahoma Health Sciences Center have found a way to use a radical new type of gene therapy to prevent blindness caused by retinitis pigmentosa, giving hope to the estimated 100,000 Americans who suffer from this debilitating disease.

The study appears in the Journal of the Federation of American Societies for Experimental Biology (FASEB).

The research, led by Muna Naash, Ph.D., at the University of Oklahoma Health Sciences Center, with collaborators in Cleveland and Buffalo, discovered a way to deliver known gene therapies directly to the light-sensitive cells affected by this disease.

The discovery already is being used to develop new treatments for another disease - [macular degeneration](#), the leading cause of blindness in the United States.

"I am thrilled about it. That's why we have been working so hard to get this as quickly as possible through the necessary experiments, so we can publish our findings and take it out to the patients," Naash said. "We hope the results of our study will be instrumental in generating a cure for the debilitating blindness associated with [retinitis pigmentosa](#) and other inherited and acquired retinal diseases. We want to give Oklahomans and others suffering from these diseases renewed independence and quality of life."

Utilizing nanoparticle technology, scientists created a microscopic capsule capable of carrying genetic therapies to their destination inside cells of the retina. The tiny delivery vehicle is being tested with a variety of gene therapies in animal models with the potential of treating several diseases from [bladder cancer](#) to diabetes. The capsules have proven very effective, carrying therapies to the designated location in the eye within 15 minutes of delivery and spreading the genetic repair message quickly to nearby cells.

"This is an incredible breakthrough in terms of being able to treat with gene therapy," said Robert E. Leonard, M.D., an ophthalmologist at the Dean McGee Eye Institute. "Outside of [gene therapy](#), we are at a loss to be able to treat these patients, so this is incredibly important research. It's breathtaking, very exciting."

Provided by University of Oklahoma

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