Gene therapy shows promise for reversing blindness

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Most causes of untreatable blindness occur due to loss of the millions of light sensitive photoreceptor cells that line the retina, similar to the pixels in a digital camera.

The remaining retinal nerve cells which are not light sensitive however remain in the eye. Samantha de Silva and colleagues (University of Oxford) used a viral vector to express a light sensitive protein, melanopsin, in the residual retinal cells in mice which were blind from retinitis pigmentosa, the most common cause of blindness in young people.

The mice were monitored for over a year and they maintained vision during this time, being able to recognise objects in their environment which indicated a high level of visual perception. The cells expressing melanopsin were able to respond to light and send visual signals to the brain. The Oxford team has also been trialling an electronic retina successfully in blind patients, but the genetic approach may have advantages in being simpler to administer.

The research was led by Professors Robert MacLaren and Mark Hankins at the Nuffield Laboratory of Ophthalmology in Oxford. Samantha de Silva, the lead author of the study said: "There are many blind patients in our clinics and the ability to give them some sight back with a relatively simple genetic procedure is very exciting. Our next step will be to start a clinical trial to assess this in patients."

The full paper, "Long-term restoration of visual function in end-stage retinal degeneration using subretinal human melanopsin gene therapy," can be read in *PNAS*.

More information: Samantha R. De Silva el al., "Long-term restoration of visual function in end-stage retinal degeneration using subretinal human melanopsin gene therapy," *PNAS* (2017). www.pnas.org/cgi/doi/10.1073/pnas.1701589114

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