

Avalanche's Novel Technology Restores Vision in Pre-Clinical Models of Blindness

June 12, 2013 1:11 AM ET

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In this week's issue of Science Translational Medicine, UC Berkeley scientists report on vector-mediated delivery to the outer retina using proprietary technology licensed by Avalanche. The paper, which appeared in this week's issue of Science Translational Medicine, describes a novel AAV variant that has the potential to greatly expand DNA delivery to the eye. Through directed evolution, David Schaffer and colleagues created millions of variations of the AAV virus, and selected variants for their ability to cross the retina. The resulting variant capsid, called 7m8, demonstrated strong transduction of photoreceptors and RPE cells following a simple intravitreal injection. The delivery was superior to other AAV capsids, and restored vision in mouse models of Xlinked retinoschisis and Leber's Congenital Amaurosis. Furthermore, the variant showed effective transduction in macaques, which closely mirrors the retinal anatomy found in humans.

Find out more from The Scientist magazine: <http://www.the-scientist.com/?articles.view/articleNo/35995/title/Genes-Get-in-Your-Eye/>

Read the journal article in Science Translational Medicine: <http://stm.sciencemag.org/content/5/189/189ra76>