

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Ophthalmic disease				
Retinitis	Halorhodopsin	<i>In vitro, ex vivo</i> and mouse studies suggest that <i>Natronomonas pharaonis</i> halorhodopsin gene therapy could help treat retinitis pigmentosa, a disease characterized by rod photoreceptor death and cone photoreceptor light insensitivity. In a mouse model of retinal degeneration, adeno-associated virus (AAV)-mediated gene delivery of halorhodopsin resensitized cones to light and improved behavioral responses to light compared with AAV-mediated delivery of a control protein. In <i>ex vivo</i> human retinas, halorhodopsin restored sensitivity to light-insensitive photoreceptors compared with no treatment. Next steps could include testing the gene therapy in additional animal models to determine its effect on photoreceptor survival.	Patent and licensing status unavailable	Busskamp, V. <i>et al. Science</i> ; published online June 24, 2010; doi:10.1126/science.1190897 Contact : Botand Roska, Friedrich Miescher Institute for Biomedical Research, Basel, Switzerland e-mail: botond.roska@fmi.ch

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