

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Ophthalmic disease				
Retinitis; ophthalmic disease	Guanylate cyclase activator 1A retina (GUCA1A)	Mouse studies suggest small hairpin RNA therapy could help treat retinitis pigmentosa (RP). In a transgenic mouse model of RP expressing mutant bovine Guca1a, subretinal injection of a recombinant adeno-associated viral (AAV) vector loaded with shRNA against the mutation increased both photoreceptor survival and visual acuity compared with injection of an AAV vector-delivered scrambled control shRNA. Planned work includes testing an AAV vector delivering shRNA against mutant mouse Guca1a in mouse models.	Unpatented; unlicensed	Jiang, L. <i>et al. Proc. Natl. Acad. Sci.</i> <i>USA</i> ; published online Oct. 31, 2011; doi:10.1073/pnas.1112758108 Contact: Wolfgang Baehr, The University of Utah, Salt Lake City, Utah e-mail: wbaehr@hsc.utah.edu

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