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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Ophthalmic disease				
Retinitis	*Retinitis pigmentosa GTPase regulator* (*RPGR*)	Canine studies suggest gene therapy could be used to correct defects in *RPGR* that lead to retinitis pigmentosa, a hereditary form of vision loss. In a canine model of retinitis pigmentosa, an adeno-associated virus (AAV) vector with a functional copy of human *RPGR* decreased photoreceptor degeneration and restored retinal function compared with no treatment. Next steps include optimizing gene expression of the construct in preparation for clinical testing. Applied Genetic Technologies Corp. has patents on the AAV vector used in the study and has a related construct in Phase I/II testing for Leber's congenital amaurosis, another hereditary retinal indication.	Patent pending; available for licensing	Beltran, W.A. *et al. Proc. Natl. Acad. Sci. USA*; published online Jan. 23, 2012; doi:10.1073/pnas.1118847109 **Contact:** Gustavo D. Aguirre, University of Pennsylvania, Philadelphia, Pa. e-mail: gda@vet.upenn.edu **Contact:** William A. Beltran, same affiliation as above e-mail: wbeltran@vet.upenn.edu

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