This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Ophthalmic disease				
Blindness	Bardet-Biedl syndrome 4 protein (BBS4)	<p>Mouse studies suggest that gene therapy could help treat blindness associated with Bardet-Biedl syndrome (BBS). In a <i>Bbs4</i>-deficient mouse model of BBS, mice had progressive death of photoreceptors and loss of retinal function compared with wild-type mice. In the model, adeno-associated viral (AAV) vector expression of <i>Bbs4</i> prevented photoreceptor death compared with empty control vector expression. Next steps could include testing the gene therapy in other models of BBS.</p> <p>SciBX 4(14); doi:10.1038/scibx.2011.407 Published online April 7, 2011</p>	Patent and licensing status unavailable	<p>Simons, D.L. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online March 28, 2011; doi:10.1073/pnas.1019222108</p> <p>Contact: David L. Simons, Baylor College of Medicine, Houston, Texas e-mail: dlsimons@bcm.edu</p>