This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Ophthalmic disease				
Blindness	Bardet-Biedl syndrome 3 (BBS3)	<p>A study in zebrafish and in mice suggests that increasing expression of the BBS3 long isoform could help treat Bardet-Biedl syndrome-associated retinal degeneration. In zebrafish, knockdown of the <i>bbs3</i> long isoform led to vision impairment compared with mock knockdown. In these zebrafish, injection with human <i>BBS3</i> long isoform mRNA rescued the vision defect, whereas injection with human <i>BBS3</i> mRNA did not. In mice, a deficiency in the <i>Bbs3</i> long isoform alone was sufficient to cause retinal abnormalities. Next steps could include evaluating the <i>BBS3</i> long isoform in additional animal models of BBS-associated retinal degeneration.</p> <p>SciBX 3(15); doi:10.1038/scibx.2010.473 Published online April 15, 2010</p>	Patent and licensing status unavailable	<p>Pretorius, P.R. <i>et al. PLoS Genet.</i>; published online March 19, 2010; doi:10.1371/journal.pgen.1000884</p> <p>Contact: Val C. Sheffield, The University of Iowa, Iowa City, Iowa e-mail: val-sheffield@uiowa.edu</p>