This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Ophthalmic disease				
Corneal transplant	Paired box 6 (PAX6)	Human cell culture and rabbit studies suggest optimizing cell culture conditions and inducing PAX6 activity could help generate transplantable limbic stem cells (LSCs). In a feeder-free, 3D cell culture system, overexpression of *PAX6* converted human skin epithelial stem cells to LSCs that differentiated into corneal epithelial cells. In a rabbit model of corneal disease, a corneal transplant of *Pax6*-expressing rabbit skin epithelial stem cells restored and maintained corneal clarity, whereas transplantation of rabbit LSCs with *Pax6* knockdown resulted in a skin-like opaque epithelium. Ongoing work includes using the culture system to expand autologous LSCs and designing a *PAX6*-specific gene therapy.	Patent application filed; available for licensing	Ouyang, H. *et al. Nature*; published online July 2, 2014; doi:10.1038/nature13465 **Contact:** Kang Zhang, University of California, San Diego, La Jolla, Calif. e-mail: kangzhang@ucsd.edu

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