This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Zinc finger nuclease- and piggyBac-mediated gene correction in human induced pluripotent stem (iPS) cells for gene therapy	<p><i>In vitro</i> and mouse studies suggest targeted gene correction in human iPS cells could be used for gene therapy to treat monogenic diseases. In cultured human iPS cells from individuals with α_1-antitrypsin (AAT; A₁AT; SERPINA1) deficiency, zinc finger nucleases and DNA piggyBac transposons corrected the A₁AT point mutation without leaving any extra genetic sequences at the correction site. In hepatocyte-like cells derived from the corrected human iPS cells, genetic abnormalities and mutant A₁AT secretion were absent, and wild-type A₁AT secretion and function was restored. In mice, the iPS cell-derived human hepatocyte-like cells colonized the liver, and there was no tumor formation. Next steps include scaling up the production of corrected iPS cells. Sangamo BioSciences Inc. has zinc finger nuclease-based compounds in clinical and preclinical testing for multiple indications.</p> <p>SciBX(42); doi:10.1038/scibx.2011.1194 Published online Oct. 27, 2011</p>	Patent and licensing status undisclosed	<p>Yusa, K. <i>et al. Nature</i>; published online Oct. 12, 2011; doi:10.1038/nature10424 Contact: Allan Bradley, Wellcome Trust Sanger Institute, Hinxton, U.K. e-mail: abradley@sanger.ac.uk</p>