This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Genetic correction of spinal muscular atrophy (SMA) neurons with a nonviral, nonintegrating vector	<p><i>In vitro</i> and mouse studies suggest genetic modification of patient-derived neurons with a nonviral, nonintegrating vector could help treat SMA. In induced pluripotent stem (iPS) cells derived from the fibroblasts of a patient with SMA, an episomal vector was used to convert the gene encoding <i>survival of motor neuron 2 centromeric</i> (SMN2) into a <i>survival of motor neuron 1 telomeric</i> (SMN1)-like gene that produced the full-length SMN protein. In a mouse model of SMA, spinal cord engraftment of neurons derived from the vector-treated human iPS cells decreased disease severity and increased lifespan and neuron engraftment compared with engraftment of cells derived from untreated iPS cells. Next steps could include testing the strategy in animal models of more advanced SMA.</p> <p>Isis Pharmaceuticals Inc. and Biogen Idec Inc. have ISIS-SMNRx, an antisense oligonucleotide modulating the splicing of SMN2 pre-mRNA, in Phase I/II testing to treat SMA.</p> <p>SciBX 6(3); doi:10.1038/scibx.2013.74 Published online Jan. 24, 2013</p>	Patent and licensing status unavailable	<p>Corti, S. <i>et al. Sci. Transl. Med.</i>; published online Dec. 19, 2012; doi:10.1126/scitranslmed.3004108</p> <p>Contact: Giacomo P. Comi, University of Milan, Milan, Italy e-mail: giacomo.comi@unimi.it</p>