This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Disease models			
Induced pluripotent stem (iPS) cell model of spinal muscular atrophy (SMA)	<p>An iPS cell model of SMA may help guide the identification and development of compounds to treat the disease. SMA is caused by a mutation in <i>survival of motor neuron 1 (SMN1)</i> that causes decreased SMN protein expression. iPS cells from a SMA patient differentiated into motor neurons that did not express SMN1 and had higher neuron degeneration than wild-type iPS cells from the patient's healthy mother. In iPS-SMA-derived neurons, SMN inducers valproic acid or tobramycin increased SMN levels compared with those seen in untreated controls. Next steps include validating the results with additional iPS cells from the same patient and in additional patients.</p> <p>Tikvah Therapeutics Inc.'s TIK-201 sodium phenylbutyrate is in Phase II testing to treat SMA.</p> <p>At least three other companies have compounds in Phase I or earlier to treat the same condition.</p> <p>SciBX 2(3); doi:10.1038/scibx.2009.118 Published online Jan. 22, 2009</p>	Patent and licensing status undisclosed	<p>Ebert, A.D. <i>et al. Nature</i>; published online Dec. 21, 2008; doi:10.1038/nature07677</p> <p>Contact: Allison Ebert, University of Wisconsin-Madison, Madison, Wis. e-mail: ebert@waisman.wisc.edu</p>