This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Huntington's disease (HD)	Huntingtin (HTT)	<p><i>In vitro</i> and mouse studies suggest stem cell therapy could help treat HD. In induced pluripotent stem (iPS) cells derived from HD patient fibroblasts, genetic replacement of the HD-associated CAG repeats in HTT generated pluripotent cells that differentiated into neuronal stem cells (NSCs) with no disease phenotype. In a mouse model of HD, transplantation of the corrected NSCs into the striatum repopulated the mouse brains with healthy human neurons. Next steps include correcting iPS cells from additional HD patients and working with the HD patient-derived iPS cells to screen for drug leads.</p> <p>SciBX 5(27); doi:10.1038/scibx.2012.707 Published online July 12, 2012</p>	Unpatented; available for licensing	<p>An, M.C. <i>et al. Cell Stem Cell</i>; published online June 28, 2012; doi:10.1016/j.stem.2012.04.026 Contact: Lisa M. Ellerby, Buck Institute, Novato, Calif. e-mail: ellerby@buckinstitute.org</p>