This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Huntington's disease (HD)	Huntingtin (HTT)	<p>Mouse and nonhuman primate studies suggest transient knockdown of <i>HTT</i> expression could help treat HD. In transgenic mouse models of HD, intracerebroventricular infusion of an antisense oligonucleotide against <i>HTT</i> mRNA decreased HTT expression and increased both motor function and survival compared with infusion of saline. The beneficial effects persisted for up to nine months after stopping treatment. In a nonhuman primate model of HD, the oligonucleotide decreased HTT expression throughout the brain compared with vehicle control. Next steps include clinical development of the anti-HTT oligonucleotide.</p> <p>Isis Pharmaceuticals Inc. collaborated in this study and has an unnamed anti-HTT oligonucleotide in preclinical development for HD.</p> <p><i>SciBX</i> 5(30); doi:10.1038/scibx.2012.790 Published online Aug. 2, 2012</p>	Patented by Isis Pharmaceuticals; available for licensing	<p>Kordasiewicz, H.B. <i>et al. Neuron</i>; published online June 21, 2012; doi:10.1016/j.neuron.2012.05.009</p> <p>Contact: Don W. Cleveland, University of California, San Diego, La Jolla, Calif. e-mail: dcleveland@ucsd.edu</p>