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 allele-selective single-strand small interfering RNA (ss-siRNA) could help treat HD. In fibroblast cell lines derived from patients with HD, several chemically modified ss-siRNAs selectively silenced mutant but not wild-type <i>HTT</i> alleles at low nanomolar IC_{50} values. In mice that heterozygously expressed mutant <i>Htt</i>, intraventricular infusion of a lead ss-siRNA decreased levels of mutant but not wild-type <i>Htt</i> protein in the frontal cortex and other brain areas compared with vehicle. Ongoing work by Isis Pharmaceuticals Inc. includes optimizing the ss-siRNAs against <i>HTT</i> and other undisclosed targets.</p> <p><i>SciBX</i> 5(36); doi:10.1038/scibx.2012.956 Published online Sept. 13, 2012</p>	Patented by Isis Pharmaceuticals; available for licensing	<p>Yu, D. <i>et al. Cell</i>; published online Aug. 31, 2012; doi:10.1016/j.cell.2012.08.002 Contact: David R. Corey, The University of Texas Southwestern Medical Center, Dallas, Texas e-mail: david.corey@utsouthwestern.edu</p>