This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Huntington's disease (HD); Machado-Joseph disease (MJD)	Huntingtin (HTT); HD; Ataxin 3 (ATXN3; MJD)	<p>Studies in human and mouse cell culture suggest that peptide nucleic acid (PNA) and locked nucleic acid (LNA) compounds could help treat HD and MJD. HD and MJD are dominant hereditary disorders caused by aberrant nucleotide repeats in the <i>HTT</i> and <i>MJD</i> genes, respectively. In fibroblast cell lines derived from patients who were heterozygous for the HD and MJD mutations, PNA and LNA antisense oligomers directed against the repeats decreased expression of mutant proteins. Human HD fibroblast cell lines or Htt transgenic mouse cells treated with oligomers targeting mutant HTT had lower levels of apoptosis than mock-treated controls. Next steps include testing candidate PNAs and LNAs in animal models of HD and MJD.</p> <p>ALN-HTT, a small hairpin RNA against HTT from Alnylam Pharmaceuticals Inc. and Medtronic Inc., is in preclinical testing to treat HD.</p> <p>SciBX 2(21); doi:10.1038/scibx.2009.887 Published online May 28, 2009</p>	Patent pending; available for licensing	<p>Hu, J. <i>et al. Nat. Biotechnol.</i>; published online May 3, 2009; doi:10.1038/nbt.1539</p> <p>Contact: David Corey, University of Texas Southwestern Medical Center, Dallas, Texas e-mail: david.corey@utsouthwestern.edu</p>