This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Amyotrophic lateral sclerosis (ALS)	<i>Chromosome 9 open reading frame 72 (C9orf72)</i>	<p>A study of patient samples suggests reducing levels of transcripts made from <i>C9orf72</i> repeat expansions could help treat ALS. GGGGCC repeat expansions in <i>C9orf72</i> are the most common cause of ALS. In frontal cortex autopsy samples and peripheral blood taken from patients with ALS that have <i>C9orf72</i> repeat expansions, RNA foci containing both sense and antisense repeats were detected that were not seen in patients without the expansions. In <i>C9orf72</i>-containing patient brain samples, antibodies detected aggregated, repeat-associated non-ATG translation products from both sense and antisense transcripts. Next steps include additional studies to understand the basic biology of the <i>C9orf72</i> repeat expansions.</p> <p>Isis Pharmaceuticals Inc. has antisense oligonucleotide candidates against <i>C9orf72</i> in preclinical development to treat ALS.</p> <p>SciBX 7(2); doi:10.1038/scibx.2014.62 Published online Jan. 16, 2014</p>	Patent pending; available for licensing	<p>Zu, T. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online Nov. 18, 2013; doi:10.1073/pnas.1315438110</p> <p>Contact: Laura P.W. Ranum, University of Florida, Gainesville, Fla. e-mail: ranum@ufl.edu</p>