This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Amyotrophic lateral sclerosis (ALS); dementia	<i>Chromosome 9 open reading frame 72 (C9ORF72)</i>	Genetic studies identified a repeat expansion in C9ORF72 that is associated with familial frontotemporal dementia (FTD) and ALS, and could help predict risk for the diseases. In a family affected by FTD and ALS, sequencing of the disease-associated chromosome 9p21 region identified a GGGGCC hexanucleotide repeat in the noncoding region of C9ORF72 that was present in all affected patients. The mutation was identified in 16 of 26 additional FTD and ALS-affected families studied, and in 59 of 696 unrelated FTD and ALS patients. In a separate study, the same repeat was identified in 46% of familial ALS patients in a Finnish population and in 21.1% of sporadic ALS patients from a group of 402 ALS cases and 478 controls. Next steps include additional patient screens to confirm the association and determine the disease mechanism.	Patent application filed for findings in the first study by the Mayo Clinic covering FTD and ALS detection; available for licensing Patent and licensing status unavailable from the NIH for findings in the second study	DeJesus-Hernandez, M. <i>et al. Neuron</i> ; published online Sept. 21, 2011; doi:10.1016/j.neuron.2011.09.011 Contact: Rosa Rademakers, Mayo Clinic Jacksonville, Jacksonville, Fla. e-mail: rademakers.rosa@mayo.edu Contact: Ian R. Mackenzie, Vancouver General Hospital, Vancouver, British Columbia, Canada e-mail: ian.mackenzie@vch.ca Renton, A.E. <i>et al. Neuron</i> ; published online Sept. 21, 2011; doi:10.1016/j.neuron.2011.09.010 Contact: Bryan J. Traynor, National Institutes of Health, Bethesda, Md. e-mail: traynorb@mail.nih.gov
SciBX 4(41); doi:10.1038/scibx.2011.1150 Published online Oct. 20, 2011				