This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Amyotrophic lateral sclerosis (ALS)	Fusion (involved in t(12;16) in malignant liposarcoma) (FUS; TLS)	<p>Gene mapping of human CNS tissue and cell and rat neuronal culture studies suggests that FUS could be targeted to treat ALS. In CNS tissues, three mutations in the <i>FUS</i> gene were linked to a familial form of ALS. In CNS tissues from three patients with ALS-linked <i>FUS</i> mutations, FUS protein showed cytoplasmic localization, which was absent in normal controls, <i>superoxide dismutase 1</i> (<i>SOD1</i>) mutant ALS patients and patients with sporadic ALS. Cultured cells or rat cortical neurons expressing FUS proteins bearing ALS-linked mutations formed cytoplasmic aggregates, whereas wild-type protein was soluble and nuclear. Next steps include studying FUS mislocalization in animal models for ALS and examining how FUS might interact with TAR DNA-binding protein 43 (TDP-43) and <i>SOD1</i>, two other genes associated with familial ALS.</p> <p>SciBX 2(10); doi:10.1038/scibx.2009.417 Published online March 12, 2009</p>	Unpatented; licensing status not applicable	<p>Vance, C. <i>et al. Science</i>; published online Feb. 27, 2009; doi:10.1126/science. 1165942</p> <p>Contact: Christopher E. Shaw, King's College London, London, U.K. e-mail: shaw@iop.kcl.ac.uk</p>