This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Amyotrophic lateral sclerosis (ALS)	TAR DNA-binding protein 43 (TDP-43)	A gene-mapping study shows that mutations in TDP-43 cause a hereditary form of ALS, suggesting that the protein could be a therapeutic target. Three separate mutations in TDP-43 were identified by sequencing the TDP-43 gene, *TARDBP*, in a family with hereditary ALS as well as in two sporadic cases. In a chick embryo model of disease, mutant forms of TDP-43 caused greater neural apoptosis and developmental delay than in wild-type embryos. Next steps include characterizing mutant TDP-43 in a mammalian model of ALS and developing targeted therapeutics. At least 20 companies are developing therapies for ALS.	Not patented; licensing status undisclosed	Sreedharan, *et al. Science*; published online Feb. 28, 2008; doi:10.1126/science.1154584 **Contact:** Christopher E. Shaw, Department of Clinical Neurosciences, King's College London, London, U.K. e-mail: chris.shaw@iop.kcl.ac.uk