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 cell culture study suggests that altering subcellular localization of TDP-43 could be a strategy for treating ALS. Cytoplasmic localization of pathological TDP-43, found as insoluble aggregates, is associated with ALS. In human embryonic kidney cells and primary mouse hippocampal neurons, expression of mutant TDP-43 that lacked a nuclear localization sequence caused TDP-43 to incorrectly localize to the cytoplasm. Wild-type TDP-43 remained in the nucleus. Next steps include testing for possible toxicity caused by TDP-43 cytoplasmic localization, examining the localization of ALS-linked TDP-43 mutant proteins and screening for compounds that alter TDP-43 localization.	Patented; available for licensing through the Marian S. Ware Center for Alzheimer's Drug Discovery Program, University of Pennsylvania School of Medicine	Winton, M.J. *et al. J. Biol. Chem.*; published online Feb. 27, 2008; doi:10.1074/jbc.M800342200 **Contact:** Virginia M.-Y. Lee, Center for Neurodegenerative Disease Research, University of Pennsylvania School of Medicine, Philadelphia, Pa. e-mail: vmylee@mail.med.upenn.edu