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)	Gene mapping of human CNS tissue and a cell culture study suggests that FUS could be targeted to treat ALS. In CNS tissues, 13 mutations in the <i>FUS</i> gene were linked to a rare familial form of ALS. In CNS tissues from a patient with one ALS-linked <i>FUS</i> mutation, FUS protein showed cytoplasmic localization, whereas healthy controls showed nuclear localization. Cultured neuroblastoma cells expressing FUS proteins with one of two ALS-linked mutations formed cytoplasmic aggregates, whereas wild-type protein was soluble and nuclear. Next steps include studying FUS mislocalization in animal models of ALS and examining how FUS might interact with TAR DNA-binding protein 43 (TDP-43) and superoxide dismutase 1 (SOD1), two other genes associated with familial ALS.	Patent pending; available for licensing from Massachusetts General Hospital	Kwiatkowski, T.J. <i>et al. Science</i> ; published online Feb. 27, 2009; doi:10.1126/science.1166066 Contact: T.J. Kwiatkowski, Jr., Massachusetts General Hospital, Charlestown, Mass. e-mail: tjkwiatkowski@partners.org Contact: R.H. Brown, Jr., University of Massachusetts, Worcester, Mass. e-mail: robert.brown@umassmed.edu
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