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)	Cell culture studies suggest oligonucleotides that induce *FUS* exon seven skipping could help prevent the accumulation of mutant FUS protein in ALS motor neurons. FUS is a DNA- and RNA-binding protein that is frequently mutated in patients with ALS and accumulates in the cytoplasm to become neurotoxic. *In vitro* and in cultured cells, wild-type FUS autoregulated its own expression by binding to exon seven of its own pre-mRNA transcript and promoting exon skipping and pre-mRNA degradation. In cells expressing mutant *FUS* variants with defective autoregulation, antisense oligonucleotides targeting a flanking splice site in the *FUS* pre-mRNA restored exon seven skipping. Next steps include optimizing the oligonucleotides. *SciBX* 6(48); doi:10.1038/scibx.2013.1394 Published online Dec. 19, 2013	Patent and licensing status unavailable	Zhou, Y. *et al. PLoS Genet.*; published online Oct. 31, 2013; doi:10.1371/journal.pgen.1003895 **Contact:** Geoffrey G. Hicks, University of Manitoba, Winnipeg, Manitoba, Canada e-mail: hicksgg@cc.umanitoba.ca

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