This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Amyotrophic lateral sclerosis (ALS)	Chromosome 9 open reading frame 72 (C9orf72)	<i>In vitro</i> studies suggest small molecules that bind C9orf72 repeats could help treat ALS in patients who carry the repeat. In human embryonic kidney cells and fibroblast-derived neuronal cells from patients who express the ALS-associated C9orf72 repeat expansion, a small molecule that binds the expanded RNA's hairpin structure decreased formation of toxic foci generated from C9orf72 repeat RNA and repeat-associated non-ATG (RAN) translation compared with vehicle. RAN protein products were detected in cerebrospinal fluid samples from 14 patients with C9orf72 but not in samples from 25 patients lacking the mutation or 5 healthy controls. Next steps include animal and toxicity studies on the small molecule.	Patent status unavailable; licensing status not applicable	Su, Z. <i>et al. Neuron</i> ; published online Aug. 14, 2014; doi:10.1016/j.neuron.2014.07.041 Contact: Matthew D. Disney, Scripps Florida, Jupiter, Fla. e-mail: disney@scripps.edu Contact: Leonard Petrucelli, Mayo Clinic, Jacksonville, Fla. e-mail: petrucelli.leonard@mayo.edu
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