This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Amyotrophic lateral sclerosis (ALS)	Ataxin 2 (ATXN2; SCA2); TAR DNA binding protein 43 (TDP-43; TARDBP)	<p>A study in yeast, flies and ALS patients suggests that antagonizing ATXN2's interaction with TDP-43 could be useful for treating the disease. TDP-43 forms intracellular aggregates in a majority of ALS cases. In a yeast model of ALS, deletion of the yeast ortholog of Atxn2 decreased the toxicity of Tdp-43 compared with that in wild-type controls. In a transgenic fly model of neurodegeneration, Atxn2 overexpression increased TDP-43 toxicity, neurodegeneration and mortality compared with normal Atxn2 expression. ALS patients with aggregate-prone mutant ATXN2 had earlier onset and more severe disease than patients with wild-type ATXN2. Next steps include testing for interactions between ATXN2 and TDP-43 and screening for small molecule modulators of TDP-43.</p> <p>SciBX 3(35); doi:10.1038/scibx.2010.1072 Published online Sept. 9, 2010</p>	Yeast screening platform used in study patented by the Whitehead Institute for Biomedical Research; licensed to FoldRx Pharmaceuticals Inc.	<p>Elden, A.C. <i>et al. Nature</i>; published online Aug. 26, 2010; doi:10.1038/nature09320</p> <p>Contact: Aaron D. Gitler, University of Pennsylvania, Philadelphia, Pa. e-mail: gitler@mail.med.upenn.edu</p>