This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Fragile X syndrome	Fragile X mental retardation 1 (FMR1); p21 protein (Cdc42 Rac)-activated kinase (PAK)	<p>Mouse studies suggest the small molecule PAK inhibitor FRAX486 could help treat fragile X syndrome. In the <i>Fmr1</i> knockout mouse model for fragile X syndrome, FRAX486 rescued dendritic spine abnormalities, whereas vehicle did not. In this mouse model, FRAX486 decreased susceptibility to sound-induced seizures, hyperactivity and repetitive behaviors compared with vehicle. Next steps could include evaluating FRAX486 and other PAK inhibitors across multiple fragile X syndrome models. Afraxis Inc. had FRAX486 in preclinical development, and its therapeutics business was acquired by Roche's Genentech Inc. unit in February. Genentech did not disclose its development plans for Afraxis' fragile X compounds.</p> <p>SciBX 6(13); doi:10.1038/scibx.2013.317 Published online April 4, 2013</p>	Patent application filed covering small molecule PAK inhibitors; licensing status unavailable	<p>Dolan, B.M. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online March 18, 2013; doi:10.1073/pnas.1219383110</p> <p>Contact: Susumu Tonegawa, Massachusetts Institute of Technology, Cambridge, Mass. e-mail: tonegawa@mit.edu</p> <p>Contact: Bridget M. Dolan, same affiliation as above e-mail: bdolan@alum.mit.edu</p>