

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Neurology	Protein phosphatase 2 (PPP2CA; PP2A)	<i>In vitro</i> and mouse studies suggest inhibiting PPP2CA in the brain could help treat Opitz syndrome, an inherited neurological disorder. <i>Midline 1 (MID1)</i> is mutated in the X-linked form of Opitz syndrome and regulates the degradation of PPP2CA. In cultured primary mouse neurons, RNAi targeting <i>Mid1</i> increased abnormal axon growth and levels of Ppp2ca compared with those seen in wild-type neurons, and knockout of <i>Ppp2ca</i> restored normal axon growth. In mice, genetic depletion of <i>Mid1</i> disrupted normal axon growth, and a microRNA targeting <i>Ppp2ca</i> restored growth. Next steps include screening for inhibitors of PPP2CA.	Findings unpatented; unavailable for licensing	Lu, T. <i>et al. Proc. Natl. Acad. Sci. USA</i> ; published online Nov. 5, 2013; doi:10.1073/pnas.1303687110 Contact: Zhi Xiong, Shanghai Institutes for Biological Sciences, Chinese Academy of Sciences, Shanghai, China e-mail: xiongzhiq@ion.ac.cn Contact: Renchao Chen, same affiliation as above e-mail: rcchen@ion.ac.cn
		SciBX 6(48); doi:10.1038/scibx.2013.1396 Published online Dec. 19, 2013		