This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Rett syndrome	Methyl CpG binding protein 2 (MECP2; RTT); microRNA-30a (miR-30a); miR-30d; miR-381; miR-495; brain-derived neurotrophic factor (BDNF)	<p>Mouse studies suggest that inhibiting miRNAs that are upregulated in the brain of Rett syndrome patients could help treat the condition. Rett syndrome is caused by a loss-of-function mutation in <i>MECP2</i>. In mice, knockout of <i>Mecp2</i> caused greater levels of miR-30a, miR-30d, miR-381 and miR-459 in the cerebella than those in the cerebella of wild-type mice. <i>In vitro</i>, the upregulated miRNAs downregulated <i>Bdnf</i>, which could lead to worsening of Rett syndrome symptoms. Next steps include identifying a way to inhibit the upregulated miRNAs in animal brains.</p> <p>SciBX 3(41); doi:10.1038/scibx.2010.1244 Published online Oct. 21, 2010</p>	Findings unpatented; potential miRNA-based therapeutics for treating Rett syndrome or related autism spectrum disorders available for licensing	<p>Wu, H. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online Oct. 4, 2010; doi:10.1073/pnas.1005595107</p> <p>Contact: Hao Wu, University of California, Los Angeles, Calif. e-mail: haowu7@gmail.com</p>