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)	<p>Mouse studies suggest <i>MECP2</i> gene therapy could help treat Rett syndrome. Mutations in MECP2 are the most frequent cause of Rett syndrome. In <i>Mecp2</i>^{-/-} mice, viral delivery of <i>Mecp2</i> yielded physiologically relevant levels of Mecp2 in the brain and restored normal neuronal soma size, and it increased survival compared with viral delivery of a control gene. In <i>Mecp2</i>^{-/-} female mice, <i>Mecp2</i> gene transfer decreased motor impairment and seizure frequency compared with transfer of a control gene. Next steps include modifying the viral delivery vector for optimal expression of the gene.</p> <p>SciBX 6(36); doi:10.1038/scibx.2013.1004 Published online Sept. 19, 2013</p>	Patent and licensing status unavailable	<p>Garg, S.K. <i>et al. J. Neurosci.</i>; published online Aug. 21, 2013; doi:10.1523/JNEUROSCI.1854-13.2013 Contact: Gail Mandel, Oregon Health & Science University, Portland, Ore. e-mail: mandelg@ohsu.edu</p>