This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Lissencephaly	Calpain; lissencephaly-1 (PAFAH1B1; LIS1; LIS2)	<i>In vitro</i> and mouse studies suggest that inhibiting calpain could help treat lissencephaly, a disease caused by a mutation in <i>LIS1</i> that results in defective neuronal migration. In mouse <i>Lis1</i> ^{+/-} cerebellar granular neurons, calpain inhibitors improved cellular migration. In <i>Lis1</i> ^{+/-} pregnant mice, intraperitoneal injection of a calpain inhibitor prevented apoptotic neuronal cell death and partially rescued impaired motor behavior. In this same model, <i>in utero</i> small hairpin RNA knockdown of calpain rescued the defective cortical layering in the offspring. Next steps include determining the effect of administering the inhibitors postnatally because lissencephaly is normally diagnosed at birth.	Findings patented; licensing status unavailable	Yamada, M. <i>et al. Nat. Med.</i> ; published online Sept. 6, 2009; doi:10.1038/nm.2023 Contact: Shinji Hirotsune, Osaka City University Graduate School of Medicine, Asahi-machi, Osaka, Japan e-mail: shinjih@med.osaka-cu.ac.jp
<i>SciBX</i> 2(35); doi:10.1038/scibx.2009.1354 Published online Sept. 10, 2009				