

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Down syndrome	Smoothened (SMO)	<p>Mouse studies suggest agonizing SMO could be useful for treating neurodevelopmental deficits in patients with Down syndrome. In a mouse model of Down syndrome, cerebellar and hippocampal development were compromised. Perinatal treatment with a Smo-agonizing compound improved cerebellar and hippocampal development and performance in motor and learning tasks. Next steps include mouse studies to identify the brain tissues most directly affected by SMO agonists and dose-ranging and teratogenicity studies.</p> <p>SciBX 6(37); doi:10.1038/scibx.2013.1036 Published online Sept. 26, 2013</p>	Unpatented; licensing status not applicable	<p>Das, I. <i>et al. Sci. Transl. Med.</i>; published online Sept. 4, 2013; doi:10.1126/scitranslmed.3005983 Contact: Roger H. Reeves, The Johns Hopkins University School of Medicine, Baltimore, Md. e-mail: reeves@jhmi.edu</p>