This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Peroxisome biogenesis disorders	Not applicable	<p><i>In vitro</i> screening assays identified compounds that could help treat Zellweger spectrum disorder (ZSD), a condition caused by mutations in enzymes of the peroxisome. High throughput screening in ZSD fibroblasts identified three compounds that restored normal peroxisome function. A second <i>in vitro</i> assay confirmed the restorative activity of the three compounds, two of which were protein kinase C (PKC) inhibitors and one that was a flavone compound, acacetin diacetate. Ongoing work includes further testing of the three hits, screening larger compound libraries and developing a knock-in mouse model of a <i>peroxisome biogenesis factor 1 (PEX1)</i> mutation that is common in peroxisome-related disorders.</p> <p>SciBX 3(12); doi:10.1038/scibx.2010.386 Published online March 25, 2010</p>	Unpatented; available for licensing	<p>Zhang, R. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online March 8, 2010; doi:10.1073/pnas.0914960107</p> <p>Contact: Nancy Braverman, McGill University, Montreal, Quebec, Canada e-mail: nancy.braverman@mcgill.ca</p>