This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Niemann-Pick Type C disease	Niemann-Pick disease, type C1 (NPC1); NPC2	<p>Studies in mice suggest that the sterol-binding agent 2-hydroxypropyl-β-cyclodextrin (CYCLO) could be useful for treating Niemann-Pick Type C disease. The lysosomal storage disease results from mutations in either <i>NPC1</i> or <i>NPC2</i> that cause cholesterol accumulation in lysosomes, leading to progressive liver disease and neurodegeneration. In mice lacking <i>npc1</i>, a single dose of CYCLO at seven days of age caused sequestered cholesterol to flow from the lysosomes to the cytosolic compartment in a variety of organs, leading to decreased expression of proinflammatory proteins in the brain and liver compared with what was seen in untreated mice. By 49 days of age, whole-body cholesterol was decreased, liver function was improved and neurodegeneration was minimized compared with that seen in untreated mice. Next steps include investigating the molecular mechanism behind CYCLO's activity.</p> <p><i>SciBX</i> 2(5); doi:10.1038/scibx.2009.197 Published online Feb. 5, 2009</p>	Unpatented; unlicensed	<p>Liu, B. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online Jan. 26, 2009; doi:10.1073/pnas.0810895106</p> <p>Contact: John Dietschy, University of Texas Southwestern Medical School, Dallas, Texas e-mail: john.dietschy@utsouthwestern.edu</p>