

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Glycosphingolipid storage disorders	Histone deacetylase (HDAC)	<i>In vitro</i> studies suggest that HDAC inhibitors could help treat the lysosomal storage disorder Niemann-Pick type C disease. In human fibroblasts with homo- or heterozygous Niemann-Pick disease type C1 (NPC1) mutations, the HDAC inhibitor panobinostat decreased cholesterol accumulation, increased NPC1 protein levels and restored cholesterol homeostasis compared with vehicle control. Next steps include testing HDAC inhibition in additional models. Panobinostat from Novartis AG is in multiple clinical trials to treat cancer.	Patent applications filed by the University of Notre Dame; university is in licensing discussions with an undisclosed startup company	Pipalia, N.H. <i>et al. Proc. Natl. Acad. Sci. USA</i> ; published online March 21, 2011; doi:10.1073/pnas.1014890108 Contact: Frederick R. Maxfield, Weill Cornell Medical College, New York, N.Y. e-mail: frmaxfie@med.cornell.edu
<p>SciBX 4(14); doi:10.1038/scibx.2011.406 Published online April 7, 2011</p>				