

### This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
<b>Endocrine/metabolic disease</b>				
Glycosphingolipid storage disorders	Not applicable	<p><i>In vitro</i> studies suggest 2-hydroxypropyl-<math>\beta</math>-cyclodextrin (HP<math>\beta</math>CD) could help treat lysosomal storage disorders. HP<math>\beta</math>CD has been used to improve drug stability and bioavailability, but there is evidence that the compound has activity against cholesterol storage disorders. In HeLa cells, HP<math>\beta</math>CD activated transcription factor EB (TFEB), which is involved in lysosomal regulation, and induced autophagy. In fibroblasts from a patient with the late infantile neonatal ceroid lipofuscinosis lysosomal storage disease, HP<math>\beta</math>CD improved clearance of ceroid lipopigment, which accumulated in patient cells, through TFEB activation and autophagy. Next steps could include testing the effects of HP<math>\beta</math>CD in additional storage disorders.</p> <p>The NIH has HP<math>\beta</math>CD in Phase I trials to treat Niemann-Pick disease type C1 (NPC1).</p> <p><b>SciBX 7(13); doi:10.1038/scibx.2014.375</b> Published online April 3, 2014</p>	Patent and licensing status unavailable	<p>Song, W. <i>et al. J. Biol. Chem.</i>; published online Feb. 20, 2014; doi:10.1074/jbc.M113.506246</p> <p><b>Contact:</b> Laura Segatori, Rice University, Houston, Texas e-mail: <a href="mailto:segatori@rice.edu">segatori@rice.edu</a></p>