This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Gaucher's disease	Glucocerebrosidase (GBA; GCase)	<p>Cell culture studies suggest that blocking degradation of mutant GCase could help treat Gaucher's disease, which is caused by a deficiency in GCase. In cultured fibroblast cell lines carrying GCase mutations, a proteasome inhibitor or small interfering RNA against an E3 ubiquitin ligase involved in GCase degradation led to greater GCase levels and enzymatic activity than no treatment or a control siRNA. Next steps include developing small molecules that target the GCase degradation pathways.</p> <p>SciBX 4(1); doi:10.1038/scibx.2011.17 Published online Jan. 6, 2011</p>	Findings unpatented; licensing status not applicable	<p>Lu, J. <i>et al.</i> <i>Proc. Natl. Acad. Sci. USA</i>; published online Nov. 22, 2010; doi:10.1073/pnas.1014376107 Contact: Zhengping Zhuang, National Institutes of Health, Bethesda, Md. e-mail: zhuangp@ninds.nih.gov Contact: Roscoe O. Brady, same affiliation as above e-mail: bradyr@ninds.nih.gov</p>