This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Gaucher's disease	Glucocerebrosidase (GBA; GCase)	<p><i>In vitro</i> studies suggest the natural compound celastrol could help treat Gaucher's disease, which is caused by loss-of-function mutations in GCase. In fibroblasts from patients with Gaucher's disease with the two most common GCase mutations, celastrol increased both GCase activity and its levels compared with no treatment. The compound functioned by inhibiting heat shock protein 90 (Hsp90)-mediated degradation of GCase. In cultured cells, celastrol increased expression of chaperone proteins that promote stability of mutant GCase compared with vehicle. Next steps could include testing celastrol in animal models of Gaucher's disease.</p> <p>Shire plc markets Vpriv velaglucerase alfa, a recombinant GCase, to treat Gaucher's disease.</p> <p>Sanofi markets Cerezyme imiglucerase and Ceredase, a recombinant GCase and alglucerase, to treat the disease.</p> <p>Pfizer Inc. markets Protalix BioTherapeutics Inc.'s Elelyso alfatriglycerase, a recombinant GCase, for Gaucher's disease.</p> <p>Several companies have compounds targeting the GCase system in Phase III or earlier development for Gaucher's disease.</p> <p>SciBX 7(3); doi:10.1038/scibx.2014.87 Published online Jan. 23, 2014</p>	Patent and licensing status unavailable	<p>Yang, C. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online Dec. 18, 2013; doi:10.1073/pnas.1321341111</p> <p>Contact: Zhengping Zhuang, National Institutes of Health, Bethesda, Md. e-mail: zhuangp@ninds.nih.gov</p> <p>Contact: Roscoe O. Brady, same affiliation as above e-mail: bradyr@ninds.nih.gov</p> <p>Contact: Chunzhang Yang, same affiliation as above e-mail: yangc2@ninds.nih.gov</p>