

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/m	etabolic disease			
Gaucher's disease	Glucosidase-β bile acid 2 (GBA2)	Mouse studies suggest inhibiting GBA2 could help treat type 1 Gaucher's disease. In a mouse model of Gaucher's disease, <i>Gba2</i> knockout resulted in decreased disease severity compared with parental controls with no <i>Gba2</i> alteration. Next steps include testing GBA2 inhibitors on animal models of Gaucher's disease. <i>SciBX</i> 7(16); doi:10.1038/scibx.2014.461 Published online April 24, 2014	Patent and licensing status undisclosed	Mistry, P.K. <i>et al. Proc. Natl. Acad. Sci.</i> USA; published online March 17, 2014 doi:10.1073/pnas.1400768111 Contact: Mone Zaidi, Icahn School of Medicine at Mount Sinai, New York, N.Y. e-mail: mone.zaidi@mountsinai.org