This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Gaucher's disease	DnaJ homolog subfamily B member 11 (DNAJB11; ERdj3); glucocerebrosidase (GBA; GCase)	<p>Mouse studies suggest inhibiting ERdj3 could help treat Gaucher's disease, a lysosomal storage disorder caused by loss-of-function mutations in <i>GCase</i>. In patient-derived fibroblasts, siRNA against ERdj3 increased the lysosomal activity of disease-associated mutant GCase and slowed its degradation compared with control siRNA. In patient-derived fibroblasts, <i>ERdj3</i> knockdown was shown to direct the mutant GCase toward the calcium-regulated chaperone calnexin (CANX). Next steps could include screening for pharmacological inhibitors of ERdj3.</p> <p>SciBX 7(37); doi:10.1038/scibx.2014.1097 Published online Sept. 25, 2014</p>	Patent and licensing status unavailable	<p>Tan, Y.L. <i>et al. Chem. Biol.</i>; published online Aug. 14, 2014; doi:10.1016/j.chembiol.2014.06.008 Contact: Jeffery W. Kelly, The Scripps Research Institute, La Jolla, Calif. e-mail: jkelly@scripps.edu</p>