This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Gaucher's disease	Receptor-interacting serine-threonine kinase 3 (RIPK3; RIP3)	<p>Mouse studies suggest inhibiting RIPK3 could help treat forms of Gaucher's disease with neurological symptoms. Enzyme replacement therapy to restore glucocerebrosidase (GBA; GCase) does not treat neurological symptoms of the disease caused by neuron loss. In a genetic mouse model of Gaucher's disease with neurological symptoms, compared with littermates without the disease, <i>Ripk3</i>, which is involved in programmed necrosis, was upregulated in brains. In a mouse model of Gaucher's disease induced by an irreversible GCase inhibitor, <i>Ripk3</i> knockout extended lifespan and improved motor coordination, and it decreased neuron loss compared with wild-type <i>Ripk3</i> expression. Next steps include developing blood brain barrier-penetrant small molecule inhibitors of the RIPK3 pathway.</p> <p>SciBX 7(5); doi:10.1038/scibx.2014.146 Published online Feb. 6, 2014</p>	Provisional patent application filed in the U.S.; available for licensing	<p>Vitner, E.B. <i>et al. Nat. Med.</i>; published online Jan. 19, 2014; doi:10.1038/nm.3449 Contact: Anthony H. Futerman, Weizmann Institute of Science, Rehovot, Israel e-mail: tony.futerman@weizmann.ac.il</p>