This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Diabetes	Sirtuin 1 (SIRT1)	<i>In vitro</i> studies identified a SIRT1 mutation that could be associated with type 1 diabetes. Genotyping and sequencing identified a leucine to proline substitution at residue 107 of SIRT1 in four family members with type 1 diabetes and one with ulcerative colitis (UC). In β cells, expression of the mutant <i>SIRT1</i> disrupted normal SIRT1 activity and increased production of cytokines, chemokines and nitric oxide compared with expression of wild-type <i>SIRT1</i>. In myoblasts isolated from healthy controls, transduction with the SIRT1 mutant caused insulin resistance. Next steps include developing SIRT1 activators to treat type 1 diabetes. Elixir Pharmaceuticals Inc.'s EX-527, an oral SIRT1 inhibitor, is in Phase II testing to treat Huntington's disease (HD). GlaxoSmithKline plc's GSK2245840 SIRT1 activator is in Phase II testing to treat psoriasis. The compound completed Phase I/II trials for type 2 diabetes, but the pharma said it is no longer pursuing that indication with the compound. Ildong Pharmaceutical Co. Ltd.'s SIRT1 activator, ID2244, is in preclinical development to treat metabolic syndrome. SciBX 6(12); doi:10.1038/scibx.2013.289 Published online March 28, 2013	Patent and licensing status unavailable	Biason-Lauber, A. <i>et al. Cell Metab.</i>; published online March 5, 2013; doi:10.1016/j.cmet.2013.02.001 Contact: Marc Y. Donath, University Hospital Basel, Basel, Switzerland e-mail: marc.donath@usb.ch