This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
α -Antitrypsin (AAT) deficiency	α_1 -Antitrypsin (AAT; A ₁ AT; SERPINA1); histone deacetylase 7 (HDAC7)	<p><i>In vitro</i> studies suggest suberoylanilide hydroxamic acid (SAHA) could help treat AAT deficiency. The Z-variant of AAT deficiency is the most severe form of the condition, which involves defective folding, trafficking and secretion of AAT from the endoplasmic reticulum. In human cell lines expressing Z-AAT, HDAC inhibitors including SAHA increased AAT trafficking from the endoplasmic reticulum compared with vehicle. In these cells, small interfering RNA knockdown of HDAC7 increased AAT stability and trafficking compared with silencing of other HDACs. Next steps could include testing SAHA in animal models of AAT deficiency.</p> <p>At least six companies have therapeutics to treat AAT deficiency in development stages ranging from preclinical to marketed.</p> <p>Merck & Co. Inc. markets Zolanza SAHA to treat cutaneous T cell lymphoma (CTCL).</p> <p>SciBX 5(40); doi:10.1038/scibx.2012.1051 Published online Oct. 11, 2012</p>	Patent and licensing status unavailable	<p>Bouchecareilh, M. <i>et al. J. Biol. Chem.</i>; published online Sept. 20, 2012; doi:10.1074/jbc.M112.404707</p> <p>Contact: William E. Balch, The Scripps Research Institute, La Jolla, Calif. e-mail: webalch@scripps.edu</p>