This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Glycosphingolipid storage disorders	Palmitoyl-protein thioesterase 1 (PPT1)	<i>In vitro</i> and mouse studies suggest a hydroxylamine derivative could help treat infantile neuronal ceroid lipofuscinosis (INCL), a lysosomal storage disorder in which loss of PPT1 function leads to lysosomal ceroid accumulation and neuronal damage. In lymphoblasts from patients with INCL, the hydroxylamine derivative <i>N</i> -(tert-Butyl) hydroxylamine (NtBuHA) depleted lysosomal ceroids. In <i>Ppt1</i> knockout mice, NtBuHA depleted lysosomal ceroids and decreased neuronal apoptosis and increased survival compared with no treatment. Next steps include carrying out IND-enabling studies of NtBuHA.	Patent application filed; available for licensing	Sarkar, C. <i>et al. Nat. Neurosci.</i> ; published online Sept. 22, 2013; doi:10.1038/nn.3526 Contact: Anil B. Mukherjee, National Institute of Child Health and Human Development, Bethesda, Md. e-mail: mukherja@exchange.nih.gov
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