

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Niemann-Pick disease	Niemann-Pick disease, type C1 (NPC1)	<p>Studies in mice suggest that compounds that increase cytosolic calcium may be useful for treating NPC1, which is characterized by an abnormal buildup of lipids and cholesterol within cells. In an NPC1^{-/-} mouse model of the disease, oral curcumin improved coat, increased weight gain and activity, lowered tremors and prolonged survival compared with what was seen in untreated controls. Curcumin is a natural product antagonist of the sarco/endoplasmic reticulum calcium transporting ATPase (SERCA). Inhibition of SERCA increases cytosolic calcium by releasing endoplasmic reticulum calcium. Next steps include finding an optimal dose of curcumin and evaluating the compound in patients with the disease.</p> <p>Recombinant adeno-associated viral vectors encoding human phosphodiesterase 1, acid lysosomal from Genzyme Corp. are in preclinical testing to treat Niemann-Pick disease.</p> <p>SciBX 1(40); doi:10.1038/scibx.2008.976 Published online Nov. 6, 2008</p>	<p>Provisional patent filed for treatments that increase cytosolic calcium to treat NPC1; available for licensing from ISIS Innovation Ltd.</p> <p>Contact: ISIS Innovation. Ltd. e-mail: innovation@isis.ox.ac.uk</p>	<p>Lloyd-Evans, E. <i>et al. Nat. Med.</i>; published online Oct. 26, 2008; doi:10.1038/nm.1876</p> <p>Contact: Frances M. Platt, University of Oxford, Oxford, U.K. e-mail: frances.platt@pharm.ox.ac.uk</p> <p>Contact: Emyr Lloyd-Evans, same affiliation as above e-mail: emyr.lloyd-evans@pharm.ox.ac.uk</p>