This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Lipodystrophy	Phosphate cytidyltransferase 1 choline- α (PCYT1A)	<p>Biochemical and cell-based studies identified PCYT1A mutants that could be targeted to treat congenital lipodystrophies. Genetic sequencing and analyses of DNA obtained from two patients with similar metabolic disease profiles identified biallelic mutations in <i>PCYT1A</i>. In cell-based biochemical analyses, primary cells expressing mutant <i>PCYT1A</i> mRNA showed lower PCYT1A protein expression and PCYT1A-mediated synthesis of phosphatidylcholine than cells expressing wild-type <i>PCYT1A</i> mRNA. In cultured mouse adipocytes, <i>Pcyt1a</i>-targeting siRNA decreased lipid formation and accumulation compared with scrambled siRNA. Next steps could include testing PCYT1A activity in other forms of metabolic disease and designing compounds to compensate for the enzyme's impaired activity.</p> <p>SciBX 7(27); doi:10.1038/scibx.2014.795 Published online July 17, 2014</p>	Patent and licensing status unavailable	<p>Payne, F. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online June 2, 2014; doi:10.1073/pnas.1408523111 Contact: David Savage, University of Cambridge, Cambridge, U.K. e-mail: dbs23@medschl.cam.ac.uk Contact: Stephen O'Rahilly, same affiliation as above e-mail: so104@medschl.cam.ac.uk Contact: Inês Barroso, Wellcome Trust Sanger Institute, Cambridge, U.K. e-mail: ib1@sanger.ac.uk</p>