This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Von Gierke's disease	Glucose-6-phosphatase catalytic subunit (G6PC; G6Pase); nuclear receptor coactivator 2 (NCOA2; SRC2); RAR-related orphan receptor- α (ROR α)	<p>Studies in mice and in cell culture suggest that increasing levels of hepatic SRC2 or RORα may help treat some forms of Von Gierke's disease, a glycogen storage disorder caused by mutations in G6PC. In mice, liver-specific knockout of <i>Src2</i> decreased hepatic levels of G6PC, caused accumulation of hepatic glycogen and increased hepatic steatosis and fasting hypoglycemia—a phenotype similar to that of Von Gierke's disease. Adenoviral overexpression of Rorα increased G6pc expression in wild-type murine hepatocytes but not in <i>Src2</i> knockout hepatocytes, suggesting that both <i>Src2</i> and Rorα regulate G6pc expression. Next steps include clarifying the role of SRC2 in hepatic signaling pathways.</p> <p>Myozyme alglucosidase alfa, a recombinant human acid α-glucosidase enzyme from Genzyme Corp., is marketed to treat Pompe's disease, a glycogen storage disorder.</p> <p>SciBX 1(45); doi:10.1038/scibx.2008.1110 Published online Dec. 18, 2008</p>	Patent disclosure filed; available for licensing from the Baylor College of Medicine Licensing Group	<p>Chopra, A.R. <i>et al. Science</i>; published online Nov. 27, 2008; doi:10.1126/science.1164847</p> <p>Contact: Bert W. O'Malley, Baylor College of Medicine, Houston, Texas e-mail: berto@bcm.tmc.edu</p>