This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Pompe's disease	Acid α -glucosidase (GAA)	<p>A mouse study suggests that lentiviral-mediated transduction of <i>GAA</i> in bone marrow cells could help treat Pompe's disease, which is caused by a deficiency in <i>GAA</i>. In <i>Gaa</i>-deficient mice that received autologous bone marrow cells containing the <i>GAA</i> gene, as compared with mice that received cells containing a control vector, high activity of the enzyme in the bone marrow and spleen led to improved disease symptoms. Next steps include optimizing the conditioning regimen and cell numbers in additional preclinical models.</p> <p>Myozyme alglucosidase alfa, a recombinant human <i>GAA</i> (rhGAA) from Genzyme Corp., is marketed to treat Pompe's disease.</p> <p>SciBX 3(17); doi:10.1038/scibx.2010.524 Published online April 29, 2010</p>	Patent and licensing status undisclosed	<p>van Til, N.P. <i>et al. Blood</i>; published online April 12, 2010; doi:10.1182/blood-2009-11-252874 Contact: Gerard Wagemaker, Erasmus University Medical Center, Rotterdam, the Netherlands e-mail: g.wagemaker@erasmusmc.nl</p>