This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Yeast cell lines for producing improved enzyme replacement therapies to treat Pompe's disease	<p>Genetically modified strains of the yeasts <i>Yarrowia lipolytica</i> and <i>Pichia pastoris</i> may be useful for producing Pompe's disease enzyme replacement therapies that have better tissue uptake than unmodified acid α-glucosidase (GAA). The yeast strains were modified to express a highly glycosylated form of human recombinant GAA, the lysosomal enzyme that is absent or dysfunctional in Pompe's disease. GAA was then purified from the yeast and treated with two bacterial glycosidase enzymes to generate GAA highly enriched in mannose-6-phosphate (M-6-P), a carbohydrate that targets GAA to the lysosome. <i>In vitro</i>, the M-6-P-enriched GAA was taken up by Pompe's disease patient fibroblasts in larger quantities than unmodified GAA. In a mouse model of Pompe's disease, the M-6-P-enriched GAA cleared heart and thigh muscle of glycogen better than unmodified GAA. Next steps carried out by Oxyrane U.K. Ltd. include animal toxicology studies. Myozyme and Lumizyme are human recombinant GAA enzyme replacement therapies marketed by Sanofi's Genzyme Corp. unit to treat Pompe's disease.</p> <p>SciBX 5(48); doi:10.1038/scibx.2012.1272 Published online Dec. 13, 2012</p>	Findings patented; licensing status undisclosed	<p>Tiels, P. <i>et al. Nat. Biotechnol.</i>; published online Nov. 18, 2012; doi:10.1038/nbt.2427 Contact: Wouter Vervecken, Oxyrane U.K. Ltd., Manchester, U.K. e-mail: wvervecken@oxyrane.com Contact: Nico Callewaert, Flanders Institute for Biotechnology (VIB), Ghent, Belgium e-mail: nico.callewaert@dmbr.vib-ugent.be Contact: Han Remaut, same affiliation as above e-mail: han.remaut@vib-vub.be</p>