This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Insulin-like growth factor-2 (IGF-2) peptide tag to improve enzyme replacement therapies to treat Pompe's disease	<p>A lysosome-targeting peptide tag may be useful for producing Pompe's disease enzyme replacement therapies that have better tissue uptake than unmodified acid α-glucosidase (GAA). A fusion protein consisting of a portion of IGF-2 linked to recombinant human GAA was expressed in and purified from mammalian cells. <i>In vitro</i>, the resulting BMN-701 (IGF-2-GAA) showed better uptake by rodent myoblasts than unmodified GAA. In a mouse model of Pompe's disease, BMN-701 led to lower levels of glycogen in heart, diaphragm and skeletal muscle than unmodified GAA. BMN-701 is in Phase I/II testing to treat Pompe's disease, with topline data expected 1Q13. 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.2012.1271 Published online Dec. 13, 2012</p>	Findings patented; licensing status undisclosed	<p>Maga, J.A. <i>et al. J. Biol. Chem.</i>; published online Nov. 27, 2012; doi:10.1074/jbc.M112.438663 Contact: Jonathan H. LeBowitz, BioMarin Pharmaceuticals Inc., Novato, Calif. e-mail: jlebowitz@bmrn.com</p>