This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Mucopoly-saccharidosis VII (MPS-VII, Sly syndrome)	β-glucuronidase (GUS)	A study in cell culture and mice suggests that a chemically modified form of GUS may have utility in treating CNS symptoms of MPS-VII. *In vitro*, wild-type GUS was chemically modified to prevent its uptake by mannose receptors and thus slow plasma clearance and increase passage across the blood-brain barrier. In murine MPS-VII models, treatment with modified GUS for 12 weeks was more effective at clearing neuronal lysosomal storage burden than treatment with the wild-type enzyme. Next steps include investigating the mechanism by which chemically modified GUS is delivered to the CNS.	Patent application filed in the U.S. for enhanced delivery of protein pharmaceuticals across the blood-brain barrier and to other resistant sites; available for licensing	Grubb, J. *et al. Proc. Natl. Acad. Sci. USA*; published online Feb. 11, 2008; doi:10.1073/pnas.0712147105 **Contact:** William S. Sly, Saint Louis University School of Medicine, St. Louis, Mo. e-mail: slyws@slu.edu