This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Mucopolysaccharidosis	α-L-iduronidase (IDUA)	Feline studies suggest liver-directed *IDUA* gene therapy could help treat mucopolysaccharidosis I, which is caused by IDUA deficiency and characterized by pathological accumulation of glycosaminoglycans (GAGs) in various tissues. In feline models of mutant IDUA MPS I, i.v. injection of a liver-tropic adeno-associated virus serotype 8 (AAV8) vector encoding feline *IDUA* and a liver-specific promoter led to increased serum IDUA levels compared with no treatment. In this model, the AAV gene therapy decreased GAG levels in the liver, myocardium, aortic valves and other peripheral tissues. Ongoing work in collaboration with ReGenX Biosciences LLC includes planning a clinical trial to evaluate i.v. delivery of the AAV8-*IDUA* gene therapy to treat MPS I. BioMarin Pharmaceutical Inc. and Sanofi market recombinant IDUA Aldurazyme laronidase to treat MPS I. ReGenX and Lysogene S.A.S. have SAF-301, an intracerebrally administered AAV10 vector encoding *N-sulfoglucosamine sulfohydrolase* (*SGSH*; *HNS*) and *sulfatase modifying factor 1* (*SUMF1*), in Phase I/II testing to treat MPS IIIA. ReGenX and Esteve S.A. have an intracerebroventricularly administered AAV9 vector encoding *SGSH* in preclinical development for MPS IIIA.	Patented by the University of Pennsylvania; licensed to ReGenX	Hinderer, C. *et al. Proc. Natl. Acad. Sci. USA*; published online Sept. 29, 2014; doi:10.1073/pnas.1413645111 **Contact:** James M. Wilson, University of Pennsylvania, Philadelphia, Pa. e-mail: wilsonjm@mail.med.upenn.edu

SciBX 7(40); doi:10.1038/scibx.2014.1180
Published online Oct. 16, 2014