This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Mucopolysaccharidosis	*N-sulfoglucosamine sulfohydrolase* (*SGSH*; *HNS*)	Animal studies suggest *SGSH* gene therapy could help treat mucopolysaccharidosis IIIA (MPS IIIA). In mouse models for MPS IIIA and in normal dogs, intracerebral injection of an adeno-associated virus serotype 9 (AAV9) vector encoding murine or canine *Sgsh* increased Sgsh activity in the brain compared with injection of empty vector. In the mouse models, the gene therapy decreased behavioral deficits and disease symptoms in peripheral organs and increased locomotor function and survival compared with empty vector. Ongoing studies include testing intracerebral delivery of an AAV9 vector–based gene therapy in animal models for MPS IIIB. Esteve S.A. has the AAV9 vector–based *SGSH* gene therapy in preclinical testing to treat MPS IIIA. Lysogene's SAF-301, an intraparenchymally administered AAV10 vector encoding human *SGSH* and *sulfatase modifying factor 1* (*SUMF1*), has completed Phase I/II testing to treat MPS IIIA. *SciBX* 6(29); doi:10.1038/scibx.2013.759 Published online Aug. 1, 2013	Patented by the Autonomous University of Barcelona and Esteve; licensed to Esteve	Haurigot, V. *et al. J. Clin. Invest.*; published online July 1, 2013; doi:10.1172/JCI66778 **Contact:** Fátima Bosch, Autonomous University of Barcelona, Bellaterra, Spain e-mail: fatima.bosch@uab.es