This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Mucopolysaccharidosis	α -L-iduronidase (IDUA)	<p>Feline studies suggest intrathecal <i>IDUA</i> gene therapy could help treat mucopolysaccharidosis I (MPS I), which is caused by a deficiency in the gene and characterized by glycosaminoglycan accumulation in various tissues. In mutant <i>IDUA</i> feline models of MPS I, intrathecal injection of an adeno-associated virus serotype 9 (AAV9) vector expressing feline <i>IDUA</i> increased IDUA levels in cerebrospinal fluid and serum and IDUA activity in the brain and spinal cord compared with no treatment. In this model, the gene therapy decreased glycosaminoglycan levels in the brain, liver, spleen, heart and lungs compared with no treatment. Ongoing work in collaboration with ReGenX Biosciences LLC includes planning a clinical trial of intrathecal AAV9/<i>IDUA</i> gene therapy to treat MPS I. BioMarin Pharmaceutical Inc. and Sanofi market the recombinant IDUA Aldurazyme laronidase to treat MPS I.</p> <p>ReGenX and Lysogene S.A.S. have SAF-301, an intracerebrally administered AAV10 vector encoding <i>N-sulfoglucosamine sulfohydrolase</i> (<i>SGSH</i>; <i>HNS</i>) and <i>sulfatase modifying factor 1</i> (<i>SUMF1</i>), in Phase I/II testing to treat MPS IIIA. ReGenX and Esteve S.A. have an intracerebroventricularly administered AAV9 vector encoding <i>SGSH</i> in preclinical development to treat MPS IIIA.</p> <p>SciBX 7(36); doi:10.1038/scibx.2014.1071 Published online Sept. 18, 2014</p>	Patented; licensed to ReGenX	<p>Hinderer, C. <i>et al. Mol. Ther.</i>; published online Aug. 26, 2014; doi:10.1038/mt.2014.135</p> <p>Contact: James M. Wilson, University of Pennsylvania, Philadelphia, Pa. e-mail: wilsonjm@mail.med.upenn.edu</p>