This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Mucopolysaccharidosis	α-L-iduronidase (IDUA)	*In vitro* and mouse studies suggest megakaryocyte-targeted gene therapy could help treat mucopolysaccharidosis I (MPS I; Hurler syndrome). In a human megakaryocytic cell line, an erythroid-targeting vector expressing *IDUA* increased IDUA protein secretion by 30-fold compared with no treatment. In MPS I mice, transplant of hematopoietic stem cells (HSCs) in which 1%–2% of cells were transfected with the *IDUA*-expressing vector increased IDUA levels in plasma and platelets to levels comparable to those observed in normal mice. In the MPS I mice, the HSC transplants decreased levels of glycosaminoglycans in liver, spleen and other organs compared with no treatment. Future studies could include testing the gene therapy in mouse models of other types of MPS. BioMarin Pharmaceutical Inc. markets Aldurazyme laronidase, a form of recombinant IDUA, to treat MPS I. Athersys Inc.'s MultiStem, allogeneic multipotent adult progenitor cells obtained from the bone marrow of healthy adult donors, is in preclinical testing to treat MPS I. ArmaGen Technologies Inc. has AGT-181, re-engineered human IDUA fused to IgG, in preclinical testing to treat MPS I. ***SciBX*** **7(7); doi:10.1038/scibx.2014.200** **Published online Feb. 20, 2014**	Patent and licensing status unavailable	Dai, M. *et al. Proc. Natl. Acad. Sci. USA*; published online Feb. 3, 2014; doi:10.1073/pnas.1323155111 **Contact:** Dao Pan, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio e-mail: dao.pan@cchmc.org **Contact:** Roscoe O. Brady, National Institutes of Health, Bethesda, Md. e-mail: bradyr@ninds.nih.gov