This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Mucopolysaccharidosis	*N*-acetylglucosaminidase-α (NAGLU); insulin-like growth factor-2 (IGF-2); mannose-6-phosphate receptor (M6P receptor)	Mouse studies suggest a NAGLU-IGF-2 fusion protein could help treat mucopolysaccharidosis IIIB and have improved properties over unfused recombinant NAGLU. In fibroblasts from patients with MPS IIIB, recombinant NAGLU fused to an M6P receptor–binding peptide of IGF-2 showed greater uptake than unfused recombinant NAGLU. In a mouse model of MPS IIIB, intracerebroventricular injections of the fusion protein decreased disease markers including heparan sulfate accumulation and hexosaminidase B (HEXB) levels compared with vehicle. Ongoing work in collaboration with BioMarin Pharmaceutical Inc. includes identifying potential markers of response to the fusion protein. BioMarin has the NAGLU-IGF-2 fusion protein used in the study (BMN 250) in preclinical development to treat MPS IIIB. Synageva BioPharma Corp. has the recombinant human NAGLU SBC-103 in preclinical development for the same indication. uniQure N.V. has AMT-110, an adeno-associated virus (AAV) vector expressing an shRNA against *apolipoprotein B-100* (*APOB-100*), in Phase I/II testing to treat MPS IIIB.	Patented by BioMarin; licensing status unavailable	Kan, S.-h. *et al. Proc. Natl. Acad. Sci. USA*; published online Sept. 29, 2014; doi:10.1073/pnas.1416660111 **Contact:** Elizabeth F. Neufeld, University of California, Los Angeles, Calif. e-mail: eneufeld@mednet.ucla.edu **Contact:** Mika Aoyagi-Scharber, BioMarin Pharmaceutical Inc., Novato, Calif. e-mail: maoyagi-scharber@bmrn.com **Contact:** Patricia I. Dickson, Los Angeles Biomedical Research Institute at Harbor–UCLA Medical Center, Torrance, Calif. e-mail: pdickson@labiomed.org

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