This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Mucolipin 1 (MCOLN1; ML4)	Mouse studies suggest enhancing ML4 activity could help treat muscular dystrophies. In mice, knockout of *Ml4* decreased muscle function and increased symptoms of muscular dystrophy compared with unaltered *Ml4* expression. In the *Mdx* mouse model of muscular dystrophy, injection of an adeno-associated virus (AAV) vector expressing human *ML4* into one gastrocnemius muscle decreased muscle tissue fibrosis and necrosis and increased repair of the muscle cell membrane (sarcolemma) compared with no injection. Next steps include testing gene therapy and small molecule approaches to enhance levels of *ML4* expression and ML4 function, respectively. *SciBX* 7(40); doi:10.1038/scibx.2014.1184 Published online Oct. 16, 2014	Patent status not applicable; unavailable for licensing	Cheng, X. *et al. Nat. Med.*; published online Sept. 14, 2014; doi:10.1038/nm.3611 **Contact:** Haoxing Xu, University of Michigan, Ann Arbor, Mich. e-mail: haoxingx@umich.edu **Contact:** Xiping Cheng, same affiliation as above e-mail: xpcheng@umich.edu