

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Latent transforming growth factor-β binding protein 4 (LTBP4)	Mouse studies suggest inhibiting cleavage of LTBP4 could help treat muscular dystrophies. In mouse models of cardiotoxin-induced muscle injury, expression of human <i>LTBP4</i> increased fibrosis and other muscular pathologies compared with no <i>LTBP4</i> expression. In a mouse model of Duchenne muscular dystrophy (DMD), expression of human <i>LTBP4</i> led to more severe muscle pathology than no <i>LTBP4</i> expression. Also in this model, an antibody that inhibited LTBP4 cleavage decreased muscle pathology compared with vehicle. Next steps could include screening for CNS-penetrant small molecule inhibitors of LTBP4 cleavage.	Patent and licensing status unavailable	Ceco, E. et al. Sci. Transl. Med.; published online Oct. 22, 2014; doi:10.1126/scitranslmed.3010018 Contact: Elizabeth M. McNally, The University of Chicago, Chicago, Ill. e-mail: emmcnall@uchicago.edu

SciBX 7(45); doi:10.1038/scibx.2014.1322 Published online Nov. 20, 2014