This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Collagen type VI $\alpha 1$ (COL6A1); beclin 1 autophagy related (BECN1; beclin1)	<p>Studies in human tissue and in mice suggest that inducing autophagy could help treat Bethlem myopathy, Ullrich congenital muscular dystrophy (UCMD) and other muscular dystrophies caused by <i>COL6A1</i> mutations. In patients with Bethlem myopathy or UCMD and in a <i>Col6a1</i>-knockout mouse model of muscular dystrophy, muscle tissues had lower levels of the proautophagic protein BECN1 than tissues from healthy patients or wild-type mouse controls. In the same knockout mice, activation of autophagy decreased muscular degeneration and increased muscle strength compared with those in controls. Planned work includes investigating the role of autophagy in other congenital muscular dystrophies.</p> <p>SciBX 3(43); doi:10.1038/scibx.2010.1296 Published online Nov. 4, 2010</p>	Patented by the University of Padua and the not-for-profit Telethon Italy; available for licensing or partnering	<p>Grumati, P. <i>et al. Nat. Med.</i>; published online Oct. 31, 2010; doi:10.1038/nm.2247</p> <p>Contact: Paolo Bonaldo, University of Padua, Padua, Italy e-mail: bonaldo@bio.unipd.it</p> <p>Contact: Marco Sandri, same affiliation as above e-mail: macro.sandri@unipd.it</p>