This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Dysferlin (DYSF); proteasome	<p>Cell culture studies suggest inhibiting proteasome-mediated degradation of DYSF could help treat muscular dystrophy. In human myoblasts with missense mutations in the <i>DYSF</i> gene, the proteasome inhibitors Velcade bortezomib and lactacystin rescued the ability to repair membrane injuries and induce myotubule formation. Next steps include proof-of-concept studies evaluating Velcade in patients with missense mutations in <i>DYSF</i>.</p> <p>Takeda Pharmaceutical Co. Ltd. and Johnson & Johnson market Velcade to treat multiple myeloma (MM) and mantle cell lymphoma (MCL).</p> <p>Lactacystin is a bacteria-derived chemical reagent that inhibits the proteasome.</p> <p>SciBX 5(9); doi:10.1038/scibx.2012.232 Published online March 1, 2012</p>	Unpatented; unavailable for licensing	<p>Azakhir, B.A. <i>et al. J. Biol. Chem.</i>; published online Feb. 8, 2012; doi:10.1074/jbc.M111.329078</p> <p>Contact: Michael Sinnreich, University Hospital Basel, Basel, Switzerland e-mail: msinnreich@uhbs.ch</p>