This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Muscleblind-like 1 (MBNL1); dystrophia myotonica protein kinase (DMPK)	<p>Studies in cell culture and in mice suggest that inhibitors of MBNL1 could help treat myotonic muscular dystrophy (MMD), a disease caused by the binding of MBLN1 to a mutant <i>DMPK</i> gene and the incorrect splicing of multiple genes. In a human cell line, the small molecule pentamidine prevented mis-splicing of two MMD type 1-associated genes. In a mouse model of MMD type 1, pentamidine partially prevented mis-splicing of two disease-associated genes but did not improve disease symptoms. Ongoing work includes identifying pentamidine analogs that could fully rescue gene mis-splicing and treat the disease.</p> <p>Insmed Inc.'s iPlex mecasermin rinfabate, an inhibitor of insulin-like growth factor-1 receptor (IGF1R; CD221), is in Phase II testing to treat MMD patients with severe insulin resistance.</p> <p>SciBX 2(40); doi:10.1038/scibx.2009.1513 Published online Oct. 15, 2009</p>	Patented by the University of Oregon; available for licensing	<p>Warf, M.B. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online Oct.12, 2009; doi:10.1073/pnas.0903234106</p> <p>Contact: J. Andrew Berglund, University of Oregon, Eugene, Ore. e-mail: aberglund@molbio.uoregon.edu</p>