This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Dystrophia myotonica-protein kinase (DMPK; DM1); RNaseH	<p>Mouse and cell culture studies identified modified antisense oligonucleotides that could help treat myotonic dystrophy type 1, which is caused by nuclear accumulation of mutant DMPK mRNA with expanded CUG repeats. In nonhuman primate fibroblast cell lines expressing DMPK mRNA, an oligonucleotide with CAG repeat sequences and an RNaseH recruitment site promoted degradation of DMPK with expanded CUG repeats and had no effect on the degradation of DMPK with normal CUG repeats. In a mouse model of myotonic dystrophy type 1, a different CAG repeat oligonucleotide decreased levels of mutant DMPK compared with a control oligonucleotide. Next steps could include developing a method to safely and peripherally deliver a CAG repeat antisense oligonucleotide.</p> <p>SciBX 5(12); doi:10.1038/scibx.2012.311 Published online March 22, 2012</p>	Patented by Isis Pharmaceuticals Inc.; licensing status unavailable	<p>Lee, J.E. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online Feb. 27, 2012; doi:10.1073/pnas.1117019109</p> <p>Contact: Thomas A. Cooper, Baylor College of Medicine, Houston, Texas e-mail: tcooper@bcm.edu</p>