This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Dystrophia myotonica protein kinase (DMPK)	<i>In vitro</i> and mouse studies identified a <i>DMPK</i> CUG repeat-binding inhibitor that could help treat myotonic dystrophy type 1 (DM1). DM1 is caused by CUG repeats in the <i>DMPK</i> mRNA that bind and sequester the splicing regulator muscleblind (MBNL1). In HeLa cells expressing CUG repeat-containing <i>DMPK</i> , an inhibitor that bound the repeats and blocked binding to MBNL1 decreased formation of ribonuclear foci and partially corrected splicing of two genes compared with no treatment. In a transgenic <i>Drosophila</i> model of DM1, the inhibitor suppressed disease phenotypes. Next steps could include testing the inhibitor in additional DM1 models and improving its potency.	Patent and licensing status unavailable	Wong, C.-H. <i>et al. J. Am. Chem. Soc.</i> ; published online April 4, 2014; doi:10.1021/ja5012146 Contact: Steven C. Zimmerman, University of Illinois at Urbana-Champaign, Urbana Ill. e-mail: sczimmer@illinois.edu Contact: Paul J. Hergenrother, same affiliation as above e-mail: hergenro@illinois.edu
SciBX 7(19); doi:10.1038/scibx.2014.563 Published online May 15, 2014				