This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Dystrophia myotonica protein kinase (DMPK); muscleblind-like 1 (MBNL1)	<i>In vitro</i> and mouse studies suggest that targeting triplet RNA repeats in the untranslated region of the <i>DMPK</i> mRNA with an antisense morpholino could help treat myotonic dystrophy type 1. RNA triplet repeats bind the splicing regulator MBNL1, leading to abnormal splicing and abnormal neuromuscular function. <i>In vitro</i> , the antisense morpholino CAG25 bound the RNA repeat, blocked formation of mRNA-MBNL1 complexes and disrupted existing complexes. In a transgenic mouse model of myotonic dystrophy, intramuscular injection of CAG25 corrected abnormal splicing and reduced myotonia compared with injection of a control antisense morpholino. Next steps include systemic delivery of CAG25 in mice and safety and toxicology studies.	Patent application filed; available for licensing through the University of Rochester	Wheller, T. <i>et al. Science</i> ; published online July 17, 2009; doi:10.1126/science.1173110 Contact: Charles A. Thornton, University of Rochester, Rochester, N.Y. e-mail: charles_thornton@urmc.rochester.edu
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