This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug delivery			
Systemic delivery of antisense oligonucleotide therapy for myotonic dystrophy type 1 (DM1)	<p>A study in mice suggests subcutaneous injection of an antisense oligonucleotide could help treat DM1. In a mouse model of DM1, subcutaneous injection of antisense oligonucleotide targeting sequences derived from DM1-associated mutant dystrophin myotonia protein kinase (DMPK; DM1) led to lower accumulation of nuclear transcripts than subcutaneous injection of saline. Antisense oligonucleotide-treated mice had better muscle function in a variety of muscle groups than saline-treated controls. Next steps include selection of a preclinical candidate that maximizes mutant DMPK1 knockdown in other mouse models of DM1.</p> <p>Partners Isis Pharmaceuticals Inc. and Biogen Idec Inc. have compounds related to this study in discovery-stage development for DM1.</p> <p>Prosensa B.V. has antisense oligonucleotides targeting DMPK in preclinical development for DM1.</p> <p>SciBX 5(31); doi:10.1038/scibx.2012.829 Published online Aug. 9, 2012</p>	<p>Patents related to this technology held by Isis Pharmaceuticals; unavailable for licensing</p>	<p>Wheeler, T.M. <i>et al. Nature</i>; published online Aug. 2, 2012; doi:10.1038/nature11362 Contact: Charles A. Thornton, University of Rochester, Rochester, N.Y. e-mail: charles_thornton@urmc.rochester.edu</p>