This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug delivery			
Peripheral drug delivery for spinal muscular atrophy (SMA)	<p>Mouse studies suggest peripheral administration of therapeutics could be useful for treating SMA. Prior studies focused on CNS delivery of SMA therapeutics. In a mouse model of severe SMA, subcutaneous administration of an antisense oligonucleotide that increases the splicing of survival of motor neuron 2 centromeric (Smn2; Smnc) mRNA stimulated production of Smn2 in both CNS and non-CNS tissues compared with administration of saline. The molecule also improved motor function and extended survival compared with saline. Intracerebroventricular (ICV) plus subcutaneous delivery of the oligonucleotide increased survival of mice compared with ICV delivery alone. Next steps include testing other Smn2-stimulating agents in less severe mouse models of SMA.</p> <p>Isis Pharmaceuticals Inc. scientists coauthored the paper. The company's ISIS-SMNRx is in preclinical development for SMA.</p> <p>SciBX(42); doi:10.1038/scibx.2011.1192 Published online Oct. 27, 2011</p>	<p>Antisense oligonucleotide used in study is patented; licensed to Isis Pharmaceuticals</p>	<p>Hua, Y. <i>et al. Nature</i>; published online Oct. 5, 2011; doi:10.1038/nature10485 Contact: Adrian R. Krainer, Cold Spring Harbor Laboratory, Cold Spring Harbor, N.Y. e-mail: krainer@cshl.edu</p>