This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Spinal muscular atrophy (SMA)	<i>Survival of motor neuron 2 centromeric (SMN2; SMNC)</i>	<p>Studies in mice suggest that targeting SMN2 with antisense oligonucleotides could help treat SMA. In a mouse model of severe SMA, a single intrathecal injection of the SMN2 RNA-binding oligonucleotide ISIS-SMNRx increased SMN protein levels, which are decreased in SMA, improved motor physiology and function and increased survival compared with injection of a control oligonucleotide. In nonhuman primates, intrathecal infusion of ISIS-SMNRx led to therapeutic concentrations of the oligonucleotide in all regions of the spinal cord. Next steps include testing ISIS-SMNRx in IND-enabling toxicology studies. Isis Pharmaceuticals Inc.'s ISIS-SMNRx, an antisense oligonucleotide modulating the splicing of SMN2, is in preclinical development to treat SMA. In 2008, Isis granted Genzyme Corp. right of first negotiation to ISIS-SMNRx, which Genzyme gave up in 2011 for undisclosed reasons.</p> <p>SciBX 4(12); doi:10.1038/scibx.2011.342 Published online March 24, 2011</p>	Patent application filed by Isis; available for partnering	<p>Passini, M.A. <i>et al. Sci. Transl. Med.</i>; published online March 2, 2011; doi:10.1126/scitranslmed.3001777</p> <p>Contact: Marco A. Passini, Genzyme Corp., Framingham, Mass. e-mail: marco.passini@genzyme.com</p>