This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Spinal muscular atrophy (SMA)	Survival of motor neuron 1 telomeric (SMN1)	<p>Studies in mice and monkeys suggest that SMN gene therapy could help treat SMA. In a mouse model of SMA, i.v. delivery of an adeno-associated virus serotype 9 (AAV9) vector encoding human SMN led to higher levels of SMN in the brain, spinal column and muscles and greater survival than no treatment. In monkeys, i.v. injection of the AAV9 vector encoding GFP led to expression of the protein in the primate CNS, demonstrating that the vector crossed the blood brain barrier and thus potentially avoided the necessity of directly injecting the gene therapy into the brain or spinal cord. Next steps include safety studies of the AAV9-SMN1 gene therapy in pigs and nonhuman primates.</p> <p>Trophos S.A.'s olesoxime (TRO19622), a small molecule with cholesterol-like structure that interacts with the mitochondrial permeability transition pore (MPTP), is in Phase IIa testing to treat SMA.</p> <p>California Stem Cell Inc.'s Motorgraft, a stem cell-derived motor neuron replacement product, is in preclinical development to treat SMA.</p> <p>Isis Pharmaceuticals Inc.'s ISIS-SMNRx, an antisense oligonucleotide modulating the splicing of pre-mRNA <i>survival of motor neuron 2 centromeric</i> (SMN2; SMNC), is in preclinical development to treat SMA.</p> <p>Paratek Pharmaceuticals Inc. has tetracycline analogs in discovery research to treat SMA.</p>	Findings patented; available for licensing from Nationwide Children's Hospital	<p>Foust, K. <i>et al. Nat. Biotechnol.</i>; published online Feb. 28, 2010; doi:10.1038/nbt.1610</p> <p>Contact: Brian Kaspar, The Ohio State University, Columbus, Ohio e-mail: KasparB@pediatrics.ohio-state.edu</p> <p>Contact: Arthur Burghes, same affiliation as above e-mail: burghes.1@osu.edu</p>
<p>SciBX 3(11); doi:10.1038/scibx.2010.350 Published online March 18, 2010</p>				