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); survival of motor neuron 2 centromeric (SMN2; SMNC)	<p>Mouse and cell culture studies identified a series of arylpiperidine-based SMN1 and SMN2 modulators that could help treat SMA. In patient fibroblasts, arylpiperidines from the series caused dose-dependent increases in SMN1 and SMN2 expression compared with vehicle. In mice, two of the arylpiperidines penetrated the blood brain barrier. Next steps include evaluating the lead arylpiperidine in mouse models of SMA and in human motor neurons.</p> <p>At least six companies have compounds in Phase II testing or earlier to treat SMA.</p> <p>SciBX 4(33); doi:10.1038/scibx.2011.941 Published online Aug. 25, 2011</p>	<p>Patent application filed; available for licensing from the NIH Office of Technology Transfer</p> <p>Contact: Steven Standley, National Institutes of Health, Bethesda, Md. phone: 301-435-4074 e-mail: sstand@mail.nih.gov</p>	<p>Xiao, J. <i>et al. J. Med. Chem.</i>; published online Aug. 5, 2011; doi:10.1021/jm200497t</p> <p>Contact: Jingbo Xiao, National Institutes of Health, Bethesda, Md. e-mail: xiaoj@mail.nih.gov</p> <p>Contact: Juan J. Marugan, same affiliation as above e-mail: maruganj@mail.nih.gov</p>