This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular atrophy	Survival of motor neuron 1 telomeric (SMN1; SMN); survival of motor neuron 2 centromeric (SMN2; SMNC)	<p><i>In vivo</i> studies identified a tetracycline derivative that could help treat spinal muscular atrophy (SMA), which is caused by aberrant levels of SMN. In two mouse models of SMA, the derivative (PTK-SMA1) increased the concentration of SMN in liver cells compared with vehicle. PTK-SMA1 also increased the frequency of the <i>Smn2</i> mRNA splice variant of SMN, which codes for the stable and functional version of the protein. Next steps could include developing analogs of PTK-SMA1 that can penetrate the blood brain barrier.</p> <p>Paratek Pharmaceuticals Inc.'s PTK-SMA1 is in preclinical development to treat SMA.</p> <p>TIK-201 sodium phenylbutyrate, a histone deacetylase (HDAC) inhibitor from Tikvah Therapeutics Inc., is in Phase II testing to treat SMA.</p> <p>Trophos S.A.'s Olesoxime, a small molecule with a cholesterol-like structure that interacts with the mitochondrial permeability transition pore (MPTP), is in Phase I testing for the indication.</p> <p>SMN1-G, a lentivector expressing human SMN from Oxford BioMedica plc, is in preclinical testing to treat SMA.</p> <p>SciBX 2(46); doi:10.1038/scibx.2009.1701 Published online Dec. 3, 2009</p>	PTK-SMA1 patented by Paratek Pharmaceuticals; licensing status undisclosed	<p>Hastings, M.L. <i>et al. Sci. Transl. Med.</i>; published online Nov. 4, 2009; doi:10.1126/scitranslmed.3000208</p> <p>Contact: Michelle L. Hastings, Rosalind Franklin University of Medicine and Science, Chicago, Ill. e-mail: michelle.hastings@rosalindfranklin.edu</p> <p>Contact: Paul Higgins, Paratek Pharmaceuticals Inc., Boston, Mass. e-mail: phiggins@paratekpharm.com</p> <p>Contact: Adrian R. Krainer, Cold Spring Harbor Laboratory, Cold Spring Harbor, N.Y. e-mail: krainer@cshl.edu</p>