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); stasimon (STAS; CG8408)	<p>Fruit fly and zebrafish studies suggest restoring SMN levels in specific neuronal subpopulations and promoting expression of the downstream protein STAS could help treat SMA. In fruit fly models of SMA, restoring <i>Smn</i> expression in proprioceptive neurons and interneurons rescued SMA-associated defects in motor neurons and muscle cells, whereas restoring <i>Smn</i> expression in motor neurons and muscle cells did not. Also in SMA fruit flies, injection of mRNA encoding human STAS decreased the SMA-associated muscular and neuronal defects compared with control mRNA injection. Next steps include studying whether other genes downstream of <i>SMN</i> also are involved in SMA and identifying specific neural circuits that are dysfunctional in the condition.</p> <p>The investigators have 4-aminopyridine (4-AP), a potassium channel blocker, in a Phase II/III trial to treat SMA.</p> <p>Ampyra dalfampridine, a sustained-release formulation of 4-AP, is marketed by Acorda Therapeutics Inc., Biogen Idec Inc. and Elan Corp. plc to improve walking ability in patients with multiple sclerosis (MS).</p> <p>SciBX 5(42); doi:10.1038/scibx.2012.1116 Published online Oct. 25, 2012</p>	Patent application filed for findings in both studies; available for licensing from Columbia Technology Ventures	<p>Imlach, W.L. <i>et al. Cell</i>; published online Oct. 12, 2012; doi:10.1016/j.cell.2012.09.011 Contact: Brian D. McCabe, Columbia University Medical Center, New York, N.Y. e-mail: brian@mccabelab.org</p> <p>Lotti, F. <i>et al. Cell</i>; published online Oct. 12, 2012; doi:10.1016/j.cell.2012.09.012 Contact: Brian D. McCabe, Columbia University Medical Center, New York, N.Y. e-mail: brian@mccabelab.org Contact: Livio Pellizzoni, same affiliation as above e-mail: lp2284@columbia.edu</p>