This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Spinal muscular atrophy (SMA)	Small conductance calcium-activated potassium channel (KCNN; SK)	<p>Cell culture and <i>Caenorhabditis elegans</i> studies suggest blocking KCNN signaling could be useful for treating SMA, which is caused by insufficient levels of survival of motor neuron (SMN) protein. The amyotrophic lateral sclerosis (ALS) drug Rilutek riluzole is being evaluated in patients who have SMA, but the molecular targets underlying its neuroprotective effects are unclear. In rat hippocampal neurons, riluzole prevented the decrease in axon outgrowth caused by small interfering RNA-mediated knockdown of SMN expression. In <i>C. elegans</i> models for SMA, loss of KCNN channels blocked the neuroprotective effects of riluzole. Next steps include evaluating the effect of riluzole on KCNN channels in additional SMA models. Sanofi markets Rilutek, a sodium channel blocker.</p> <p>SciBX 6(15); doi:10.1038/scibx.2013.369 Published online April 18, 2013</p>	Unpatented; licensing status not applicable	<p>Dimitriadi, M. <i>et al. J. Neurosci.</i>; published online April 10, 2013; doi:10.1523/JNEUROSCI.1536-12.2013 Contact: Anne C. Hart, Brown University, Providence, R.I. e-mail: anne_hart@brown.edu</p>