This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Spinal muscular atrophy (SMA)	<i>Survival of motor neuron 2 centromeric (SMN2; SMNC)</i>	<i>In vitro</i> studies suggest that increasing endogenous levels of the SMN protein in motor neurons could help treat SMA. In SMA, the SMN protein encoded by <i>SMN2</i> is highly unstable and present at abnormally low levels in motor neurons, leading to neurological dysfunction and muscular atrophy. Cell culture studies showed that SMN displayed an amino acid sequence that led to its rapid degradation in the proteasome. In the same cells, proteasome inhibitors blocked degradation of SMN, leading to higher cellular levels of SMN than those in untreated cells. Next steps include screening for small molecules that increase SMN levels in order to treat disease by preventing the protein's degradation in the proteasome.	Findings covered by multiple patents; available for licensing from the University of Pennsylvania Center for Technology Transfer	Cho, S. & Dreyfuss, G. <i>Genes Dev.</i> ; published online March 1, 2010; doi:10.1101/gad.1884910 Contact: Gideon Dreyfuss, University of Pennsylvania School of Medicine, Philadelphia, Pa. e-mail: gdreyfuss@hhmi.upenn.edu
<i>SciBX</i> 3(11); doi:10.1038/scibx.2010.351 Published online March 18, 2010				