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)	<i>In vitro</i> and mouse studies suggest that a codon-optimized variant of the <i>SMN1</i> gene could help treat SMA, a disease caused by absent or nonfunctional SMN protein. In human cell culture, vector-mediated expression of the codon-optimized <i>SMN1</i> variant led to higher levels of SMN than expression of the wild-type <i>SMN1</i> gene. In one-day-old SMA mouse models, systemic injection of a vector expressing codon-optimized <i>SMN1</i> led to widespread expression of the SMN protein, which extended lifespan and decreased muscle weakness compared with injection of a vector encoding for a control protein. Next steps include preclinical safety studies in nonhuman primates.	Unpatented; unavailable for licensing	Valori, C.F. <i>et al. Sci. Transl. Med.</i> ; published online June 9, 2010; doi:10.1126/scitranslmed.3000830 Contact: Mimoun Azzouz, The University of Sheffield, Sheffield, U.K. e-mail: m.azzouz@sheffield.ac.uk
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