This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Musculoskeletal disease	<i>Docking protein 7 (DOK7)</i>	<p>Mouse model studies suggest <i>DOK7</i> gene therapy could help treat neuromuscular diseases characterized by small neuromuscular junctions (NMJs), such as limb-girdle myasthenia and muscular dystrophy. In mouse models of Emery-Dreifuss muscular dystrophy and <i>Dok7</i>-mutant myasthenia, an adeno-associated viral (AAV) vector encoding <i>DOK7</i> led to NMJ enlargement and increased muscle strength and survival compared with a control vector or no treatment. Next steps include testing AAV-<i>DOK7</i> gene therapy in other neuromuscular disease models and in large animals.</p> <p><i>SciBX</i> 7(39); doi:10.1038/scibx.2014.1159 Published online Oct. 9, 2014</p>	Patented by Tokyo Medical and Dental University; available for licensing	<p>Arimura, S. <i>et al. Science</i>; published online Sept. 19, 2014; doi:10.1126/science.1250744 Contact: Yuji Yamanashi, The University of Tokyo, Tokyo, Japan e-mail: yyamanas@ims.u-tokyo.ac.jp</p>