This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Ryanodine receptor 1 (RYR1); FK506 binding protein 4 (FKBP1A; calstabin-1)	<p>Studies in mice suggest that stabilizing the Ca²⁺ channel RYR1 to prevent Ca²⁺ leakage could help treat muscular dystrophy. In <i>mdx</i> mouse skeletal muscle, RYR1 S-nitrosylation was increased and the Ca²⁺ channel stabilizer calstabin-1 was depleted, resulting in Ca²⁺ leakage compared with what was seen in age-matched, wild-type controls. Also in the <i>mdx</i> mice, the calstabin-1 depletion inhibitor S107 decreased spontaneous Ca²⁺ release events and Ca²⁺-activated enzyme activity and increased forelimb grip strength, downhill running ability and voluntary exercise compared with what was seen in controls. Next steps include developing a Ca²⁺ channel-stabilizing compound with better pharmacokinetics than S107.</p> <p>At least nine companies have therapeutics to treat muscular dystrophy in clinical and preclinical development.</p> <p>SciBX 2(7); doi:10.1038/scibx.2009.289 Published online Feb. 19, 2009</p>	Findings patented by Columbia University; Armgo Pharma Inc. holds worldwide, exclusive rights to IP for muscular dystrophy; available for sublicensing	<p>Bellinger, A. <i>et al. Nat. Med.</i>; published online Feb. 8, 2009; doi:10.1038/nm.1916</p> <p>Contact: Andrew R. Marks, Columbia University College of Physicians and Surgeons, New York, N.Y. e-mail: arm42@columbia.edu</p>