

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Nemaline myopathy	Actin- α 1 skeletal muscle (ACTA1); actin- α cardiac muscle 1 (ACTC1)	A study in mice suggests that activation of ACTC1 expression could help treat nemaline myopathy, a type of muscular dystrophy. In mice lacking <i>Acta1</i> , a model of nemaline myopathy, muscle-specific expression of human <i>ACTC1</i> prevented lethality and improved muscle structure and function. Next steps could include developing transcriptional activators of <i>ACTC1</i> expression and testing their effect on muscle function in <i>Acta1</i> knockout mice.	IP position currently under review by The University of Western Australia; licensing status undisclosed	Nowak, K. <i>et al. J. Cell Biol.</i> ; published online May 25, 2009; doi:10.1083/jcb.200812132 Contact: Kristen J. Nowak, The University of Western Australia, Perth, Western Australia, Australia e-mail: knowak@cyllene.uwa.edu.au
<p>SciBX 2(24); doi:10.1038/scibx.2009.981 Published online June 18, 2009</p>				