

### This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
<b>Musculoskeletal disease</b>				
Musculoskeletal disease	Myostatin (GDF-8)	<p>Studies in macaques suggest that follistatin gene therapy could help treat muscle diseases. Follistatin is a naturally occurring inhibitor of myostatin, a protein that prevents growth of muscle tissue. In cynomolgus macaques, quadriceps injection of an adeno-associated virus expressing follistatin led to greater muscle strength and size than no treatment. Also in the monkeys, long-term intramuscular delivery did not result in toxicity or organ damage. Toxicology and biodistribution studies are ongoing, and the researchers plan to start Phase I testing in 2010 to treat muscle diseases characterized by weakening of the quadriceps muscle.</p> <p>Acceleron Pharma Inc.'s ACE-031, a myostatin inhibitor, is in Phase I testing to treat muscular dystrophy. Amgen Inc.'s myostatin inhibitor, AMG-745, is in Phase I testing to treat muscular atrophy.</p> <p><b>SciBX 2(47); doi:10.1038/scibx.2009.1733</b> Published online Dec. 10, 2009</p>	<p>Multiple patent claims filed by The Research Institute at Nationwide Children's Hospital covering use of follistatin gene therapy to improve muscle mass and muscle function to treat musculoskeletal and neuromuscular disorders; Institute is seeking to partner and/or license the technology</p>	<p>Kota, J. <i>et al. Sci. Transl. Med.</i>; published online Nov. 11, 2009; doi:10.1126/scitranslmed.3000112 <b>Contact:</b> Brian K. Kaspar, The Ohio State University, Columbus, Ohio e-mail: <a href="mailto:KasparB@pediatrics.ohio-state.edu">KasparB@pediatrics.ohio-state.edu</a> <b>Contact:</b> Jerry R. Mendell, The Research Institute at Nationwide Children's Hospital, Columbus, Ohio e-mail: <a href="mailto:jerry.mendell@nationwidechildrens.org">jerry.mendell@nationwidechildrens.org</a></p>