This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Fukutin (FKTN)	<p><i>In vitro</i> and mouse studies suggest antisense oligonucleotides that restore FKTN mRNA splicing could help treat Fukuyama congenital muscular dystrophy (FCMD), which is caused by an insertion in the <i>FKTN</i> gene that decreases expression of the normal protein. In mice expressing the human mutant <i>FKTN</i> gene and in lymphoblasts and myotubes from FCMD patients, antisense oligonucleotides targeting splicing acceptor and enhancer sites increased FKTN levels and restored function compared with a control oligonucleotide. Next steps could include testing in additional animal models.</p> <p>SciBX 4(42); doi:10.1038/scibx.2011.1184 Published online Oct. 27, 2011</p>	Patent and licensing status undisclosed	<p>Taniguchi-Ikeda, M. <i>et al. Nature</i>; published online Oct. 5, 2011; doi:10.1038/nature10456 Contact: Tatsushi Toda, Kobe University Graduate School of Medicine, Kobe, Japan e-mail: toda@med.kobe-u.ac.jp</p>