This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Duchenne muscular dystrophy (DMD)	Dystrophin (DMD)	<p>Mouse studies suggest clustered, regularly interspaced short palindromic repeats (CRISPR)-mediated genome editing could be used to help treat DMD. In a mouse model of DMD, CRISPR genome editing of early zygotes using a short guide RNA targeting a point mutation in the <i>Dmd</i> gene created mosaic animals with 2% 100% corrected <i>Dmd</i> DNA. In the CRISPR-edited mice, the extent of muscle phenotype correction and the percentage of dystrophin-expressing muscle fibers showed that even mosaic correction of the <i>Dmd</i> mutation was capable of rescuing the disease phenotype. Next steps include using the technique in adult animals.</p> <p>Prosensa Holding N.V. has PRO052, an antisense oligonucleotide targeting <i>DMD</i>, in preclinical testing for muscular dystrophy.</p> <p>SciBX 7(36); doi:10.1038/scibx.2014.1075 Published online Sept. 18, 2014</p>	Patent application filed; available for licensing	<p>Long, C. <i>et al. Science</i>; published online Aug. 14, 2014; doi:10.1126/science.1254445</p> <p>Contact: Eric N. Olson, The University of Texas Southwestern Medical Center, Dallas, Texas e-mail: eric.olson@utsouthwestern.edu</p>