This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Duchenne muscular dystrophy (DMD)	Dystrophin (DMD)	*In vitro* and mouse studies suggest the ryanodine receptor (RyR) inhibitor dantrolene could enhance the effects of antisense oligonucleotides to treat Duchenne muscular dystrophy. In primary human and mouse muscle cells with disease-associated *DMD* mutations, dantrolene increased the exon-skipping effect of antisense oligonucleotides compared with vehicle control. In the *mdx* mouse model of Duchenne muscular dystrophy, dantrolene plus antisense oligonucleotides increased DMD expression and muscle function compared with the antisense alone or antisense plus vehicle. Next steps include dose-optimization studies of the combination therapy. Prosensa B.V. and GlaxoSmithKline plc have PRO51, an antisense oligonucleotide that induces the skipping of exon 51 of *DMD*, in Phase III trials to treat Duchenne muscular dystrophy. Sarepta Therapeutics Inc. has eteplirsen, a phosphorodiamidate morpholino oligomer (PMO) targeting exon 51, in Phase IIb testing to treat Duchenne muscular dystrophy.	Patents pending; licensing status undisclosed	Kendall, G.C. *et al. Sci. Transl. Med.*; published online Dec. 12, 2012; doi:10.1126/scitranslmed.3005054 **Contact:** M. Carrie Miceli, University of California, Los Angeles, Calif. e-mail: cmiceli@ucla.edu **Contact:** Stanley F. Nelson, same affiliation as above e-mail: snelson@ucla.edu

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