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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular atrophy	Survival of motor neuron 1 telomeric (SMN1; SMN); survival of motor neuron 2 centromeric (SMN2; SMNC)	Studies in mice suggest that an antisense oligonucleotide targeting the *SMN2* gene could increase levels of SMN and help treat spinal muscular atrophy. In humans, two genes—*SMN1* and *SMN2*—can encode the SMN protein. Spinal muscular atrophy is caused by mutations in or loss of *SMN1* paired with an *SMN2* that produces mostly an alternatively spliced, nonfunctional SMN transcript, which results in very low levels of functional SMN. In *Smn1* knockout mice expressing a homolog of the human *SMN2* gene, an antisense oligonucleotide targeting the alternative splicing site on *SMN2* restored functional SMN levels in brain and spinal cord to 50% of those in healthy mice. The mice also had increased body weight and partially improved motor defects compared with mice that received control antisense oligonucleotide. Next steps include testing the antisense oligonucleotides in other neurological conditions. At least nine companies have compounds in clinical and preclinical testing to treat muscular atrophy. *SciBX* 2(25); doi:10.1038/scibx.2009.1018 Published online June 25, 2009	Provisional patent applications filed for use of splice-modulating oligonucleotides in neurological indications including AML and epilepsy—spinal muscular atrophy not covered under these applications; the splice-modulating oligonucleotides covered by the filed patent applications are available for licensing	Williams, J. *et al. J. Neurosci.*; published online June 19, 2009; doi:10.1523/JNEUROSCI.0950-09.2009 **Contact:** Gordon J. Lutz, Drexel University College of Medicine, Philadelphia, Pa. e-mail: glutz@drexelmed.edu