SciBX
Science-Business eXchange

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	Bridging integrator 1 (BIN1)	Studies in patient samples and in mice suggest restoring BIN1 function could help treat myotonic dystrophy. In muscle cells from patients with myotonic dystrophy, misregulated skipping of exon 11 of *BIN1* mRNA correlated with disease severity. In mice, expression of an oligonucleotide that induced *Bin1* exon 11 skipping decreased muscle strength compared with expression of a vector control. Next steps include identifying drugs that could correct the splicing defect. *SciBX* 4(23); doi:10.1038/scibx.2011.661 Published online June 9, 2011	Unpatented; licensing status undisclosed	Fugier, C. *et al. Nat. Med.*; published online May 29, 2011; doi:10.1038/nm.2374 **Contact:** Nicolas Charlet-Berguerand, University of Strasbourg, Illkirch, France e-mail: ncharlet@igbmc.fr