This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Fragile X syndrome	Fragile X mental retardation 1 (FMR1)	*In vitro* studies suggest inhibiting the binding of CGG repeat–containing *FMR1* transcripts to the *FMR1* promoter could help treat fragile X syndrome. Fragile X syndrome is caused by a CGG trinucleotide expansion of more than 200 repeats that are adjacent to the *FMR1* promoter and that silence *FMR1*, but the mechanism of silencing is unknown. In human embryonic stem cells from patients with fragile X syndrome, knockdown of *FMR1* mRNA or a small molecule that binds the CGG-containing mRNA blocked silencing of the gene. In the patient cells, the CGG repeat region of *FMR1* mRNA directly bound the promoter region of *FMR1* to induce silencing. Next steps include assessing the safety of the small molecule.	Patent applications filed covering the small molecule; available for licensing	Colak, D. *et al. Science*; published online Feb. 28, 2014; doi:10.1126/science.1245831 **Contact:** Samie R. Jaffrey, Weill Cornell Medical College, New York, N.Y. e-mail: srj2003@med.cornell.edu

SciBX 7(13); doi:10.1038/scibx.2014.378
Published online April 3, 2014

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