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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Autism spectrum disorder (ASD)	μ-Opioid receptor (OPRM1; MOR); corticotropin-releasing factor receptor 1 (CRHR1; CRFR1)	Studies in mice suggest antagonizing OPRM1 and/or CRHR1 could help treat a form of ASD caused by duplication of the *methyl CpG binding protein 2* (*MECP2*; *RTT*) gene. In a mouse model of *Mecp2* duplication, $Crhr1^{+/-}$ mice or wild-type mice treated with CRHR1 antagonists had lower anxiety than $Crhr1^{+/+}$ controls. In the same model, $Oprm1^{+/-}$ mice had fewer social behavior abnormalities than $Oprm1^{+/+}$ controls. Next steps could include identification of CRHR1 and OPRM1 inhibitors for clinical development.	Patent and licensing status undisclosed	Samaco, R.C. *et al. Nat. Genet.*; published online Jan. 8, 2012; doi:10.1038/ng.1066 **Contact:** Huda Y. Zoghbi, Baylor College of Medicine, Houston, Texas e-mail: hzoghbi@bcm.edu

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