

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Autism; neurology; seizures	Mammalian target of rapamycin (mTOR; FRAP; RAFT1)	Studies in mice suggest that inhibiting mTOR may be useful for treating PTEN-associated neurological disorders, including certain cases of autism and seizures. Mice with <i>Pten</i> mutations show progressive development of brain enlargement that correlates with abnormal behavior. In <i>Pten</i> mutant mice, rapamycin significantly lowered brain-to-body ratios compared with those seen in vehicle-treated controls (p <0.05). Rapamycin also significantly lowered anxiety behaviors and seizure frequency and duration compared with what was seen using vehicle control (p <0.05). Next steps could include identifying the protein targets in the mTOR pathway responsible for the disease pathologies. Bapamune sirolimus, a rapamycin non-	Patent and licensing status unavailable	Zhou, J. <i>et al. J. Neurosci.</i> ; published online Feb. 11, 2009; doi:10.1523/JNEUROSCI.5685- 08.2009 Contact: Luis F. Parada, University of Texas Southwestern Medical Center, Dallas, Texas e-mail: luis.parada@utsouthwestern.edu

calcineurin antagonist immune suppressant from Wyeth, is marketed to prevent transplant rejection.

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