



This week in therapeutics

| Indication | Target/marker/ pathway | Summary | Licensing status | Publication and contact information |
|---|--|--|--|--|
| Neurology | | | | |
| Autism; obsessive compulsive disorder (OCD) | FK506 binding protein 1A, 12kDa (FKBP1A; FKBP12) | Studies in mice suggest that increasing FKBP12 activity in some regions of the brain could help treat autism and OCD. Conditional knockout of <i>Fkbp12</i> in mice increased hippocampal long-term potentiation but also caused behavioral alterations such as greater contextual fear memory and perseveration, which are typical traits of OCD-like behavior. Molecular analysis showed that FKBP12 generated its behavioral effects via modulation of mammalian target of rapamycin (mTOR)-mediated protein synthesis. Further studies are necessary to identify compounds that mimic FKBP12 activity without interfering with other FKBP12 targets such as ryanodine receptors or calcineurin. At least nine companies have therapeutics targeting mTOR signaling in development stages ranging from preclinical to marketed for various nonneurological indications. | Findings unpatented; mice available for licensing | Hoeffer, C. et al. Cell; published onlin Dec. 10, 2008; doi:10.1016/j.neuron.2008.09.037 Contact: Eric Klann, New York University, New York, N.Y. e-mail: eklann@cns.nyu.edu |
| | | SciBX 2(1); doi:10.1038/scibx.2009.24 Published online Jan. 8, 2009 | | |