This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Epilepsy	NMDAR; NMDA receptor NR2A subtype (GRIN2A; NR2A)	Human genetic and cell culture studies suggest modulating NMDARs could be useful for treating certain forms of epilepsy. Exome sequencing of patients with epilepsy by three independent teams found *de novo* and inherited mutations in *GRIN2A* in up to 20% of patients who have idiopathic focal epilepsy, acquired epileptic aphasia, continuous spike and waves during slow-wave sleep syndrome, benign epilepsy with centrotemporal spikes and other variants of epilepsy-aphasia syndromes. In cell culture, several disease-associated *GRIN2A* mutations caused higher NMDAR ion channel activity than wild-type *GRIN2A*. Next steps include screening for compounds that normalize the function of GRIN2A with disease-associated mutations. Mnemosyne Pharmaceuticals Inc. has GRIN2A-selective NMDAR modulators in preclinical development for schizophrenia, depression and autism. Insero Health Inc. has the NMDAR modulator huperzine A (INS001) in Phase I testing to treat drug-resistant epilepsy. At least 20 companies have nonselective NMDAR modulators in development or on the market for various neurological indications. *SciBX* 6(35); doi:10.1038/scibx.2013.968 Published online Sept. 12, 2013	Findings from all three studies unpatented; licensing status not applicable	Lesca, G. *et al. Nat. Genet.*; published online Aug. 11, 2013; doi:10.1038/ng.2726 **Contact:** Pierre Szepetowski, Aix-Marseille University, Marseille, France e-mail: pierre.szepetowski@inserm.fr Carvill, G.L. *et al. Nat. Genet.*; published online Aug. 11, 2013; doi:10.1038/ng.2727 **Contact:** Heather C. Mefford, University of Washington, Seattle, Wash. e-mail: hmefford@u.washington.edu **Contact:** Ingrid E. Scheffer, The University of Melbourne, Melbourne, Victoria, Australia e-mail: scheffer@unimelb.edu.au Lemke, J.R. *et al. Nat. Genet.*; published online Aug. 11, 2013; doi:10.1038/ng.2728 **Contact:** Sarah von Spiczak, Kiel University, Kiel, Germany e-mail: s.vonspiczak@pedneuro.uni-kiel.de **Contact:** Holger Lerche, University of Tuebingen, Tuebingen, Germany e-mail: holger.lerche@uni-tuebingen.de