This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Epilepsy	NMDA receptor NR1 subtype (GRIN1; NR1); GRIN2B (NR2B); GABA _A receptor α_1 (GABRA1); GABA _A receptor β_3 (GABRB3)	Human genome sequencing studies suggest modulating GABA _A receptors and NMDARs could help treat some patients with pediatric epilepsy. Exome sequencing of 264 patients with infantile spasms and Lennox-Gastaut syndrome and their parents identified <i>de novo</i> mutations in a variety of genes including <i>GABRB3</i> , <i>GABRA1</i> , <i>NR1</i> and <i>NR2B</i> . Next steps include characterizing the effects of these mutations on GABA _A receptor and NMDAR function in cell culture and testing pharmacological agents that modulate receptor activity. H. Lundbeck A/S markets Onfi clobazam, a GABA _A receptor agonist, to treat Lennox-Gastaut syndrome. SciBX 6(38); doi:10.1038/scibx.2013.1068 Published online Oct. 3, 2013	Unpatented; licensing status not applicable	Epi4K Consortium & Epilepsy Phenome/Genome Project. <i>Nature</i> ; published online Aug. 11, 2013; doi:10.1038/nature12439 Contact: David B. Goldstein, Duke University, Durham, N.C. e-mail: d.goldstein@duke.edu