

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Fragile X syndrome	Ras; MAP kinase 3 (MAPK3; ERK-1); MAPK1 (ERK-2)	<p>Studies in mice suggest lovastatin could help treat epilepsy in patients with fragile X syndrome. In hippocampus slices from a mouse model for fragile X syndrome, lovastatin decreased Ras-ERK-1/ERK-2 pathway signaling, abnormally high protein synthesis and epilepsy-associated activity bursts compared with vehicle. Lovastatin also decreased seizures. Next steps include dose ranging studies and developmental timing studies to identify a therapeutic window for statin treatment in patients with fragile X syndrome.</p> <p>The generic lovastatin is an HMG-CoA reductase inhibitor marketed to treat dyslipidemia and coronary artery disease (CAD).</p> <p>Seaside Therapeutics Inc.'s arbaclofen (STX-209), a GABA_B receptor antagonist, is in Phase III testing for fragile X syndrome.</p> <p>Novartis AG's AFQ056, a metabotropic glutamate receptor subtype 5 (mGluR5; GRM5) antagonist, is in Phase III trials for fragile X syndrome.</p> <p>SciBX 6(6); doi:10.1038/scibx.2013.145 Published online Feb. 14, 2013</p>	Patent pending; available for licensing	<p>Osterweil, E.K. <i>et al. Neuron</i>; published online Jan. 23, 2013; doi:10.1016/j.neuron.2012.01.034</p> <p>Contact: Mark F. Bear, Massachusetts Institute of Technology, Cambridge, Mass. e-mail: mbear@mit.edu</p>