This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Parkinson's disease (PD)	Vesicular monoamine transporter 2 (SLC18A2; VMAT2); dopa decarboxylase (DDC; AADC); calcium channel voltage-dependent T type- α 1G subunit (CACNA1G; Cav3.1)	<p>Studies in cultured mouse neurons suggest that agonizing VMAT2, or antagonizing AADC or Cav3.1, could be useful in treating PD. In murine ventral midbrain cultures treated with the dopamine precursor L-dopa, overexpression of Vmat2 decreased cytosolic dopamine and protected neurons from L-dopa-mediated neurotoxicity compared with mock-treated controls. Inhibition of Aadc and Cav3.1 also lowered cytoplasmic dopamine levels and increased cell survival in neurons treated with L-dopa compared with controls. Previously, it was thought that agonizing AADC, which converts L-dopa to dopamine, would be beneficial in PD. Next steps include devising methods to decrease levels of α-synuclein, a cellular protein that is thought to react with cytoplasmic dopamine to cause neurotoxicity in PD.</p> <p>Genzyme Corp. has an adeno-associated virus (AAV)-driven AADC gene therapeutic in Phase I for PD.</p> <p>SciBX 2(20); doi:10.1038/scibx.2009.841 Published online May 21, 2009</p>	Unpatented; licensing status not applicable	<p>Mosharov, E.V. <i>et al. Neuron.</i>; published online April 30, 2009; doi:10.1016/j.neuron.2009.01.033</p> <p>Contact: David Sulzer, Columbia University Medical Center, New York, N.Y.</p> <p>e-mail: ds43@columbia.edu</p>