

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Various				
Parkinson's disease (PD); Gaucher's disease (GD)	α -Synuclein (SNCA); glucocerebrosidase (GBA; GCCase)	<p><i>In vitro</i>, mouse and human sample studies suggest increasing GCCase expression could help treat PD. In cultured human neurons expressing PD-associated SNCA, GCCase knockout increased accumulation of toxic SNCA compared with GCCase expression. In brain tissue from a GD mouse model and in human postmortem GD brains, PD-associated SNCA levels were greater than those in samples from healthy controls, suggesting pathological feedback. Next steps could include increasing GCCase activity in preclinical models of PD.</p> <p>Cerezyme imiglucerase, a GCCase enzyme replacement therapy from the Genzyme Corp. unit of Sanofi, is marketed to treat GD.</p> <p>SciBX 4(27); doi:10.1038/scibx.2011.771 Published online July 14, 2011</p>	Findings patented; available for licensing	<p>Mazzulli, J.R. <i>et al. Cell</i>; published online June 23, 2011; doi:10.1016/j.cell.2011.06.001</p> <p>Contact: Dimitri Krainc, Massachusetts General Hospital and Harvard Medical School, Charlestown, Mass. e-mail: krainc@mgh.harvard.edu</p>