

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Glycosphingolipid storage disorders	Galactosidase β 1 (GLB1); ganglioside GM1 (GM1)	<p>Feline studies suggest intracranial delivery of adeno-associated viral (AAV) vectors encoding GLB1 could help treat GM1 gangliosidosis. GM1 gangliosidosis is an autosomal recessive lysosomal storage disorder caused by a GLB1 deficiency that results in accumulation of GM1 in neuronal tissues and leads to progressive neurodegeneration and death. In a feline model of GM1 gangliosidosis, bilateral intracranial injection of AAV1 or AAVrh8 vectors encoding feline GLB1 into the thalamus and deep cerebellar nuclei resulted in near-normal GLB1 activity in the CNS and decreased both GM1 levels in CNS tissues and neuromuscular impairments compared with no treatment. In the treated felines, mean survival was over 38 months versus 5 months for untreated controls. Next steps include running safety studies in mice and nonhuman primates and preparing an IND submission to the FDA.</p> <p>SciBX 7(18); doi:10.1038/scibx.2014.528 Published online May 8, 2014</p>	Patent application filed; licensing status unavailable	<p>McCurdy, V.J. <i>et al. Sci. Transl. Med.</i>; published online April 9, 2014; doi:10.1126/scitranslmed.3007733</p> <p>Contact: Douglas R. Martin, Auburn University, Auburn, Ala. e-mail: martidr@auburn.edu</p>