This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Adeno-associated virus 9 (AAV9)-based gene delivery across the blood-brain barrier (BBB)	<p>Studies in mice suggest that AAV9 vectors could be useful for delivering gene therapy across the BBB to adult astrocytes and neonatal neurons. In neonatal mice prior to BBB closure, facial injection of an AAV9 vector expressing green fluorescent protein (GFP) resulted in fluorescent labeling of neurons throughout most brain regions. In adult mice after BBB closure, tail vein injection of AAV9-GFP led to limited neuronal labeling but significant astrocyte labeling throughout the CNS. Next steps include determining how AAV9 vectors bypass the BBB and developing delivery strategies for neurodegenerative diseases such as amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy. At least six companies have AAV-based gene therapy vectors in development stages ranging from preclinical to Phase I/II to treat a range of diseases.</p> <p>SciBX 2(2); doi:10.1038/scibx.2009.75 Published online Jan. 15, 2009</p>	U.S. provisional patent application filed; available for worldwide licensing	<p>Foust, K. <i>et al. Nat. Biotechnol.</i>; published online Dec. 21, 2008; doi:10.1038/nbt.1515</p> <p>Contact: Brian Kaspar, Nationwide Children's Hospital, Columbus, Ohio e-mail: Brian.Kaspar@nationwidechildrens.org</p>