This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Gene therapy for Leber's hereditary optical neuropathy (LHON)	Cell culture and mouse studies suggest a mitochondria-targeted transgene could be useful for treating LHON. The majority of patients with LHON have mutations in NADH dehydrogenase subunit 4 (ND4), a mitochondrial protein involved in energy production. In a cell culture model of LHON, transfection with an adeno-associated virus (AAV) vector bearing the *ND4* gene increased mitochondrial function compared with transfection using a nontransgenic control vector. In a mouse model of LHON, the *ND4*-bearing AAV vector increased mitochondrial function and decreased vision loss compared with a control vector. Next steps include optimizing the viral expression vector. Santhera Pharmaceuticals Holding AG's Catena idebenone (SNT-MC17) is under review in Europe to treat LHON.	Patented; available for licensing	Yu, H. *et al. Proc. Natl. Acad. Sci. USA*; published online April 20, 2012; doi:10.1073/pnas.1119577109 **Contact:** John Guy, University of Miami Miller School of Medicine, Miami, Fla. e-mail: jguy@med.miami.edu

SciBX 5(19); doi:10.1038/scibx.2012.508
Published online May 10, 2012