This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Combined knockdown and replacement of disease-associated proteins	<p>Knockdown and transgenic replacement of disease-associated proteins could help treat misfolded protein diseases. An adenoviral vector was designed to carry a small hairpin RNA targeting mutant α_1-antitrypsin (AAT; A_1AT; <i>SERPINA1</i>) as well as a gene expressing wild-type AAT. In a mouse model of AAT deficiency caused by AAT mutation, injection of the vector increased wild-type gene expression and decreased mutant gene expression compared with control vector injection. Next steps include establishing long-term efficacy data followed by toxicology and biodistribution studies.</p> <p><i>SciBX</i> 4(34); doi:10.1038/scibx.2011.975 Published online Sept. 1, 2011</p>	Unpatented; available for licensing	<p>Li, C. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online Aug. 15, 2011; doi:10.1073/pnas.1109522108 Contact: R. Jude Samulski, The University of North Carolina at Chapel Hill, Chapel Hill, N.C. e-mail: rjs@med.unc.edu Contact: Chengwen Li, same affiliation as above e-mail: chengwen@med.unc.edu</p>