This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Liver-specific <i>Factor VIII</i> gene therapy for immune tolerance induction in hemophilia	Studies in canines suggest that liver-specific delivery of the genes that code for the factor VIII light and heavy chains could help treat hemophilia patients resistant to protein replacement therapy. Patients with hemophilia can develop neutralizing antibodies against the factor VIII-based protein replacement therapy, thus decreasing efficacy. In hemophilic canines with neutralizing antibodies against factor VIII, liver-specific delivery of the genes that code for the factor VIII light and heavy chains eliminated factor VIII antibodies from serum and improved disease symptoms compared with baseline. Next steps could include running longer-term safety and efficacy studies to evaluate the robustness of the response to gene therapy.	Patent and licensing status unavailable	Finn, J.D. <i>et al. Blood</i> ; published online Sept. 29, 2010; doi:10.1182/blood-2010-06-288001 Contact: Valder R. Arruda, University of Pennsylvania, Philadelphia, Pa. e-mail: arruda@email.chop.edu
<i>SciBX</i> 3(40); doi:10.1038/scibx.2010.1222 Published online Oct. 14, 2010			