This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Clustered, regularly interspaced short palindromic repeats (CRISPR)-based genome editing platform to treat genetic liver disease	Mouse studies suggest CRISPR-based genome editing could be used to treat tyrosinemia type I (TTI), a fatal disease resulting from mutation of <i>fumarylacetoacetate hydrolase</i> (FAH) and accumulation of toxic metabolites. A compound that acts upstream of FAH called 2-(2-nitro-4-trifluoromethylbenzoyl)-1,3-cyclohexanedione (NTBC) can keep <i>Fah5981SB</i>-mutant mice alive. In a <i>Fah5981SB</i> mouse model of hereditary TTI, tail vein injection of a <i>Fah</i>-correcting, single-stranded DNA (ssDNA) donor, a CRISPR-associated 9 (Cas9)-expressing ssDNA and a <i>Fah</i>-targeting single-guide RNA restored <i>Fah</i> mRNA to up to 36% of wild-type levels, resulted in widespread patches of <i>Fah</i>⁺ hepatocytes and prevented weight loss upon NTBC withdrawal. Next steps include testing the CRISPR platform in large-animal studies and developing delivery systems. SciBX 7(15); doi:10.1038/scibx.2014.442 Published online April 17, 2014	Multiple patent applications filed; licensing discussions in progress	Yin, H. <i>et al. Nat. Biotechnol.</i>; published online March 30, 2014; doi:10.1038/nbt.2884 Contact: Daniel G. Anderson, Massachusetts Institute of Technology, Cambridge, Mass. e-mail: dgander@mit.edu