This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Targeted gene editing of human hematopoietic stem cells (HSCs)	<p>Targeted gene editing of human HSCs could be useful for treating genetic diseases such as X-linked severe combined immunodeficiency (SCID-X1). The approach uses mRNA electroporation and integrase-defective lentiviral vectors to deliver zinc finger nucleases (ZFNs) and donor DNA templates into CD34⁺ human umbilical cord blood cells, and it uses targeted integration of a GFP cassette into <i>adeno-associated virus integration site 1</i> (AAVS1) or <i>IL-2 receptor γ-chain</i> (CD132; <i>IL2RG</i>). In a mouse model of SCID-X1, the approach restored the ability to reject allogeneic human breast cancer cells. In HSCs isolated from a patient with SCID-X1, the strategy was used to replace the disease-causing, mutant <i>IL2RG</i> with a functional copy of the gene. Next steps could include assessing the long-term safety of the targeted gene-editing approach in animal models.</p> <p>SciBX 7(25); doi:10.1038/scibx.2014.752 Published online June 26, 2014</p>	Patent and licensing status unavailable	<p>Genovese, P. <i>et al. Nature</i>; published online May 28, 2014; doi:10.1038/nature13420 Contact: Luigi Naldini, San Raffaele Scientific Institute, Milan, Italy e-mail: luigi.naldini@hsr.it</p>