This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
High throughput assay to identify compounds that cause nonsense codon read-through	<p>A high throughput transcription and translation assay could be useful for identifying compounds to treat diseases caused by nonsense mutations. Five compounds identified by the <i>in vitro</i> screen had a lower EC₅₀ value and less toxicity than previously identified compounds that allowed read-through of the mutations. In two cell cultures with disease-causing nonsense mutations, treatment with the best lead compounds increased the expression of mutation-containing transcripts and resulted in functional protein compared with treatment with earlier compounds or no treatment. Next steps include further screening as well as testing the lead compounds in animal models of nonsense codon-associated diseases.</p> <p>PTC Therapeutics Inc. and Genzyme Corp. have the nonsense codon-suppressing compound Ataluren (formerly PT124) in Phase III trials to treat nonsense mutation-mediated cystic fibrosis (CF) and in Phase IIb trials to treat Duchenne/Becker muscular dystrophy (DMD/BMD) due to nonsense mutations.</p> <p>SciBX 2(40); doi:10.1038/scibx.2009.1520 Published online Oct. 15, 2009</p>	Compounds and methods patented; available for licensing	<p>Du, L. <i>et al. J. Exp. Med.</i>; published online Sept. 21, 2009; doi:10.1084/jem.20081940</p> <p>Contact: Richard A. Gatti, University of California, Los Angeles, Calif. e-mail: rgatti@mednet.ucla.edu</p> <p>Contact: Liutao Du, same affiliation as above e-mail: Ldu@mednet.ucla.edu</p>