This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Site-specific modification of disease-related genes with triplex-forming peptide nucleic acids (PNAs)	*In vitro* studies suggest that triplex-forming PNAs might be useful for correcting disease-associated base-pair aberrations and treating a variety of genetically based diseases such as β-thalassemia. PNAs designed to target a single base-pair modification on the *hemoglobin*-β (*HBB*) gene induced a dose-dependent, sequence-dependent and cell-cycle stage–dependent modification of the site. The PNAs also modified aberrant *HBB* in human primary hematopoietic progenitor cells. Further alterations are necessary to increase the technique's efficacy to get enough gene correction for therapeutic effects. AVI BioPharma Inc. has an antisense morpholino oligonucleotide targeting the aberrant splice site of the *hemoglobin*-α2 (*HBA2*) gene in preclinical testing for thalassemia. Cangene Corp. markets Ferriprox deferiprone, an iron chelating agent to treat thalassemia, and Novartis AG has Exjade deferasirox (ICL670), also an iron chelating agent, in Phase III testing for the same indication.	Findings patented by Yale University; technology licensed by Helix Therapeutics LLC; available for licensing from Yale or Helix	Chin, J. *et al. Proc. Natl. Acad. Sci. USA*; published online Aug. 18, 2008; doi:10.1073/pnas.0711793105 **Contact:** Peter M. Glazer, Yale University School of Medicine, New Haven, Conn. e-mail: peter.glazer@yale.edu