This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Hematology				
Anemia	Fanconi anemia complementation group A (FANCA); FANCD2	<p>Studies in human cells suggest that induced pluripotent stem (iPS) cells carrying wild-type versions of <i>FANCA</i> or <i>FANCD2</i> could treat Fanconi anemia, an inherited disorder that predisposes individuals to acute myelogenous leukemia (AML) and other cancers. In skin cells from Fanconi anemia patients, a retroviral vector containing four transcription factors—<i>Oct4</i>, <i>Sox2</i>, <i>Klf4</i> and <i>c-Myc</i>—was used to reprogram somatic cells to a pluripotent state. Reprogrammed cells that also expressed normal FANCA or FANCD2 were able to differentiate into disease-free hematopoietic progenitor cells. Next steps could include the development of retrovirus-free methods for reprogramming somatic cells into iPS cells and genetically correcting the iPS cells.</p> <p>SciBX 2(23); doi:10.1038/scibx.2009.942 Published online June 11, 2009</p>	Patent and licensing status unavailable	<p>Raya, Á. <i>et al. Nature</i>; published online May 28, 2009; doi:10.1038/nature08129</p> <p>Contact: Juan Carlos Izpisua Belmonte, The Salk Institute for Biological Studies, La Jolla, Calif. e-mail: belmonte@salk.edu or izpisua@cmrb.eu</p>