This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Hematology				
Thalassemia	Not applicable	<p>Studies in human cell culture suggest that induced pluripotent stem (iPS) cells could help treat β-thalassemia in infants. iPS cells derived from a β-thalassemia patient's skin fibroblasts via expression of four transcription factors (<i>Oct4</i>, <i>Sox2</i>, <i>Klf4</i> and <i>c-Myc</i>) were able to differentiate into hematopoietic cells following treatment with hematopoietic cell-inducing growth factors. Human amniotic fluid cells or chorionic villus cells were also reprogrammed into iPS cells using the same four transcription factors. Next steps could include testing whether the small tissue samples used for prenatal diagnosis of β-thalassemia would be a perinatal, autologous iPS cell-based therapeutic option for β-thalassemia.</p> <p>HemaQuest Pharmaceuticals' short-chain fatty acid derivative HQK-101 is in Phase II testing to treat β-thalassemia.</p> <p>Errant Gene Therapeutics LLC's Thalagen, an autologous cell therapy of hematopoietic stem cells transduced with a lentiviral vector encoding the human <i>hemoglobin-β</i> gene, is in preclinical testing to treat β-thalassemia.</p> <p>SciBX 2(22); doi:10.1038/scibx.2009.912 Published online June 4, 2009</p>	Patent and licensing status unavailable	<p>Ye, L. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online May 25, 2009; doi:10.1073/pnas.0904689106</p> <p>Contact: Yuet Wai Kan, University of California, San Francisco, Calif. e-mail: yw.kan@ucsf.edu</p>