This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Hematology				
Sickle cell disease; β -thalassemia	B cell CLL/ lymphoma 11A (zinc finger protein) (BCL11A)	<p><i>In vitro</i> studies suggest that blocking <i>BCL11A</i> expression could increase levels of fetal hemoglobin and potentially help treat β-thalassemia or sickle cell disease. In cultured primary adult erythroid cells, downregulation of <i>BCL11A</i> led to higher levels of fetal hemoglobin than those seen in control cells that expressed normal levels of <i>BCL11A</i>. Chromatin immunoprecipitation studies showed that BCL11A affects <i>globin</i> gene regulation by binding to several sites within the β-<i>globin</i> gene cluster. Next steps include determining the effects of <i>in vivo</i> downregulation of BCL11A on fetal hemoglobin levels and disease severity.</p> <p>SciBX 2(2); doi:10.1038/scibx.2009.59 Published online Jan. 15, 2009</p>	Patent and licensing status unavailable	<p>Sankaran, V. <i>et al. Science</i>; published online Dec. 4, 2008; doi:10.1126/science.1165409 Contact: Stuart Orkin, Children's Hospital Boston, Boston, Mass. e-mail: stuart_orkin@dfci.harvard.edu</p>