This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Hematology				
Neutropenia	Growth factor independent 1 transcription repressor (GFI1); microRNA-196b (miRNA-196b); miRNA-21	<i>In vitro</i> studies suggest that miRNA-21 and miRNA-196b could be targeted to help treat severe congenital neutropenia. Bone marrow cells from severe congenital neutropenia patients contained higher levels of miRNA-21 and miRNA-196b than did cells from healthy controls. miRNA-21 and miRNA-196b were also upregulated in bone marrow cells deficient in GFI1, a zinc-finger protein whose loss is associated with severe congenital neutropenia. Overexpression of miRNA-21 and miRNA-196b blocked myelopoiesis, the main process that causes severe congenital neutropenia. Next steps include validating the findings in animal models. SciBX 2(11); doi:10.1038/scibx.2009.452 Published online March 19, 2009	Patent pending; available for licensing	Velu, C. <i>et al. Blood</i> ; published online March 10, 2009; doi:10.1182/blood-2008-11-190215 Contact: H. Leighton Grimes, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio e-mail: lee.grimes@cchmc.org