This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Hematology				
Hemophilia A	Factor VIII (FVIII); CCAAT/enhancer binding protein (C/EBP)- ϵ (CEBPE; CHOP)	<p>Cell culture and mouse studies suggest that antioxidants and CHOP inhibitors could be useful as adjuvants for helping to treat hemophilia A. In CHO cells engineered to overexpress coagulation factor FVIII, antioxidants increased the secretion of properly folded FVIII and lowered oxidative stress-induced apoptosis compared with what was seen in untreated control cells. In a mouse model of hemophilia, mice fed the antioxidant butylated hydroxyanisole and injected with a gene vector encoding FvIII had higher FvIII secretion and lower apoptosis in the liver than mice that received a mock control vector. Also in mice, <i>Chop</i> knockout had the same effect on FvIII secretion and apoptosis as antioxidant treatment. The researchers are now designing screening methods to identify potential CHOP inhibitors.</p> <p>Baxter International Inc., Bayer AG, Biotest AG, CSL Ltd., Talecris Biotherapeutics Inc. and Wyeth all market recombinant FVIII products to treat hemophilia A.</p> <p>Oxford Biomedica plc has an FVIII gene-based therapy in preclinical development to treat hemophilia A.</p> <p>SciBX 1(42); doi:10.1038/scibx.2008.1020 Published online Nov. 20, 2008</p>	Unpatented; licensing status undisclosed	<p>Malhotra, J. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published online Nov. 3, 2008; doi:10.1073/pnas.0809677105</p> <p>Contact: Randal J. Kaufman, University of Michigan Medical Center, Ann Arbor, Mich. e-mail: kaufmanr@umich.edu</p>