This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Hematology				
Hemophilia	Factor VIII	<p>Mouse and nonhuman primate studies suggest an adeno-associated virus (AAV) encoding an optimized recombinant human factor VIII could help treat hemophilia A. In wild-type mice, tail vein injection of an AAV encoding the optimized factor VIII led to 10-fold higher expression of the factor than injection of vectors encoding a nonoptimized factor VIII variant. In a mouse model for hemophilia A, injection of the optimized vector decreased tail clip-induced blood loss compared with saline injection. In nonhuman primates, peripheral vein delivery of the vector resulted in stable expression of the factor. Next steps could include evaluating the vector in nonhuman primate models for hemophilia.</p> <p>SciBX 6(9); doi:10.1038/scibx.2013.217 Published online March 7, 2013</p>	Patent and licensing status unavailable	<p>McIntosh, J. <i>et al. Blood</i>; published online Feb. 20, 2013; doi:10.1182/blood-2012-10-462200 Contact: Amit C. Nathwani, UCL Cancer Institute, University College London, London, U.K. e-mail: a.nathwani@ucl.ac.uk</p>