

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Hematology				
Hemophilia	Factor IX	<p>Mouse studies suggest lentivirus-mediated delivery of a hyperfunctional mutant factor IX could help treat hemophilia. In a mouse model of hemophilia B, low doses of lentiviral vectors encoding an optimized hyperfunctional R338L factor IX mutant led to a 15-fold gain in factor IX activity, and greater factor IX protein levels and less blood loss in a tail-clipping assay than vectors encoding a wild-type or nonoptimized factor IX. None of the tested vectors induced immunogenicity or antibodies against factor IX. Next steps include testing the strategy in larger animal models, including in canines.</p> <p>At least six companies have recombinant factor IX or delivery methods for the coagulation factor in development stages ranging from preclinical to marketed to treat hemophilia.</p> <p>SciBX 5(42); doi:10.1038/scibx.2012.1113 Published online Oct. 25, 2012</p>	Patent status unavailable; available for licensing	<p>Cantore, A. <i>et al. Blood</i>; published online Oct. 4, 2012; doi:10.1182/blood-2012-05-432591 Contact: Thierry VandenDriessche, Free University of Brussels, Brussels, Belgium e-mail: thierry.vandendriessche@vub.ac.be</p>