

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Infectious disease				
HIV/AIDS	CC chemokine receptor 5 (CCR5; CD195)	A study in human cell culture and in mice suggests that CCR5-deficient hematopoietic stem cells (HSCs) could be useful for treating HIV infection. Human HSCs transfected with a zinc finger nuclease targeting the <i>CCR5</i> gene matured into a variety of immune cell types when transplanted into mice. In a humanized mouse model of HIV infection, transplantation of those HSCs led to expansion of HIV-resistant CCR5-deficient immune cells and undetectable viral titer compared with no treatment. Next steps include clinical testing of the technique in AIDS patients with lymphoma who require a bone marrow transplant. Sangamo BioSciences Inc. has SB728, a zinc finger nuclease targeting <i>CCR5</i> in mature CD4 ⁺ cells, in a Phase I trial to treat HIV/AIDS. Benitec Ltd. has a lentiviral vector encoding three forms of anti-HIV RNA including a ribozyme targeting CCR5 in Phase I testing for HIV/AIDS.	patented by Sangamo BioSciences; additional patent and licensing information undisclosed	Holt, N. <i>et al. Nat. Biotechnol.</i> ; published online July 2, 2010; doi:10.1038/nbt.1663 Contact: Paula Cannon, University of Southern California, Los Angeles, Calif. e-mail: pcannon@usc.edu
		SciBX 3(27); doi:10.1038/scibx.2010.830		

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