Engraftment and Persistence of CCR5 Gene-Edited Hematopoietic Stem Cells in a Nonhuman Primate Model of HIV/AIDS

Chris Peterson

Hans-Peter Kiem Lab

Cell and Gene Therapy for HIV Cure 2015

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Summary for Community

- Key Question: This research addresses whether gene editing is feasible in an animal model (nonhuman primates) that most closely approximates an HIV-infected patient.
- Key Findings: We can gene-edit stem cells and safely transplant them into nonhuman primates. This is a strong argument for the safety and further evaluation of the procedure in human patients.
- Importance: Advances in gene editing are important not just for HIV patients (targeting human genes that permit infect, or could better help block it), but for patients with other genetic diseases where "corrected" genes could have therapeutic benefit.
- Cure: An important host gene used by HIV (called CCR5) is mutated in our gene-edited cells, and these cells should resist infection. We are therefore seeding a source of infection-resistant cells in our animals, which should predominate and eventually lead to cure.
- Why we should be excited: This work is exciting because gene editing has never been demonstrated before in a large animal model, but rather, only in select strains of mice. The fact that the procedure works in nonhuman primates states much more clearly that it should work in humans as well.