Docket #: S23-412

One-Time Delivery of Genetically Engineered Stem Cells Enable Long-Term Secretion of Therapeutic Antibodies for Treatment of HIV-1

Stanford researchers have engineered hematopoietic stem cells to provide long-term secretion of chosen therapeutic antibodies, eliminating the need of repeated dosing for delivery.

Thirty nine million people worldwide are living with HIV infection. Autologous transplantation of specific hematopoietic stem and progenitor cells (HSPCs) is the only known cure for HIV-1 infection. However, this treatment is extremely limited due to the rarity of specific matched donors, the morbidities associated with allogeneic transplantation, and the prevalence of HIV-1 strains resistant to select gene knockout treatments alone.

To circumvent these limitations, Stanford researchers genetically engineered HSPCs that secrete antibodies against various targets, including but not limited to HIV, PCSK9, and TNF-alpha. The genetically engineered HSPCs can be delivered via a one-time therapy through autologous transplantation. This technology could be used to develop autologous hematopoietic stem cell transplantation therapies for the long-term delivery of therapeutic antibodies for various diseases. The engineered cells also have the potential to improve current commercial treatment strategies that address long-term control of HIV-1 infection; A disease that currently requires lifetime administration of antiretroviral therapy.

Stage of research
Proof of concept

Applications

- Secrete antibodies against various targets (i.e. HIV, PCSK9, and TNF-alpha)
- Autologous hematopoietic stem cell transplantation therapies
- Research tool for control of HIV-1
- Development of antibody treatment therapies

Advantages

- Long-term secretion/sustained delivery of chosen therapeutic antibodies without repeated dosing
- Can express multiple antibodies simultaneously
- Limited comparable cell-based products currently on the market

Publications

- Feist et al. Combining Cell-Intrinsic and -Extrinsic Resistance to HIV-1 By Engineering Hematopoietic Stem Cells for CCR5 Knockout and B Cell Secretion of Therapeutic Antibodies , BioRxiv Preprint, March 09, 2024.

Patents

- Published Application: WO2025188676

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