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IN VIVO GENE THERAPY FOR SCID-X1

In Vivo Gene Therapy for X-linked Severe Combined Immunodeficiency

Brief Description of Technology

In vivo gene therapy using foamy viral vectors with a therapeutic gene to treat X-linked severe combined immunodeficiency (SCID-X1).

BUSINESS OPPORTUNITY
Exclusive license
Sponsored research

TECHNOLOGY TYPE
Gene therapy
Vectors

STAGE OF DEVELOPMENT
Preclinical *in vivo*

PATENT INFORMATION
Provisional patent pending

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Technology Overview

X-linked severe combined immunodeficiency disease (SCID-X1) is an inherited disorder that typically results in death from infections usually before 1 year of age if not treated. Current treatments are hematopoietic cell transplantation (HCT) or *ex vivo* hematopoietic stem and progenitor cell (HSPC) gene therapy. The Kiem lab has improved upon the limitations of current treatments by developing an *in vivo* gene therapy using foamy virus vector delivery to treat SCID-X1. This method employs the use of a 2nd generation vector to drive expression of therapeutic genes and mobilization of HSCs to improve delivery to target cells. The canine model of SCID-X1 showed markedly increased kinetics and clonal diversity of lymphocyte reconstitution resulting in greater thymopoiesis than existing approaches.

Applications

- Treatment for SCID-X1

Advantages

- Simple, cost effective procedure – direct delivery through injection
- Less or no genotoxic conditioning required
- Improved kinetics and clonal diversity of lymphocyte reconstitution

Market Overview

SCID-X1 affects approximately 1 in 50,000 to 75,000 newborns in the US. The best treatment option is HCT with an HLA-matched donor. However, a suitable donor is not always available and significant complications can occur when a haplo-identical transplant donor is used. The current direct cost of transplant ranges from $100,000 if the infant was treated before 3.5 months to over $450,000 if older. *Ex vivo* gene therapy is being clinically investigated as a treatment option, however, the success have been limited and typically requires genotoxic conditioning in order to allow for engineered cells to engraft. There is a significant need for an off-the-shelf treatment that eliminates the need for genotoxic conditioning and does not require extensive GMP cell manufacturing.