**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).

**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.