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GENE THERAPY PLATFORM

Universal Gene Delivery Platform for Targeting of Hematopoietic Stem Cells

Brief Description of Technology

Gold nanoparticle delivery system developed to safely genetically engineer blood stem cells at a novel safe harbor loci.

BUSINESS OPPORTUNITY
Exclusive license
Non-exclusive license
Sponsored research
Start-up

TECHNOLOGY TYPE
Platform technology
Gene therapy
HSC therapy
Reserach tool

STAGE OF DEVELOPMENT
Preclinical *in vitro*

PATENT INFORMATION
Patent pending

INVESTIGATORS
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Technology Overview

Gene therapy, the delivery of a gene into hematopoietic stem cells (HSCs), has dramatically improved the outcome and quality of life for a variety of diseases including primary immunodeficiencies and hemoglobinopathies. Current gene therapy uses engineered retroviruses to integrate therapeutic genes into the DNA of purified blood cells. However, treatment is limited by the expense and inefficiency of therapeutic retrovirus production. Moreover, all retroviruses carry a genotoxic risk. Dr. Adair and her team at Fred Hutch have developed a novel platform technology that has the potential to bypass the drawbacks of existing approaches. Her novel platform technology is a non-toxic gold nanoparticle fully loaded with all of the molecules necessary to deliver gene(s) to a safe harbor loci.

Applications

- *Ex vivo* or *in vivo* gene delivery to blood cells, including stem cells

Advantages

- Retrovirus-free gene delivery platform to avoid off-target integration
- Plug and play universal platform that allows for customization
- Direct administration to patients
- Efficient delivery with ideal toxicity profile

Market Overview

There is tremendous potential for hematopoietic stem cell (HSC) and progenitor (CD34+) cell gene therapy for many diseases. Gene therapy in blood cells is in clinical trials for over 30 disease representing a global burden of over 50 million patients. The markets for cell therapy and cell therapy manufacturing are expected to exhibit an annual growth rate of over 40% in the next 10 years. As the market grows, innovation in manufacturing and delivery is required to make cell therapy more cost effective, scalable, and offer a safe universal gene transfer platform.