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METHOD TO ISOLATE, EXPAND, AND MANIPULATE HEMATOPOIETIC STEM CELLS

Selection and Use of Hematopoietic Stem Cells for Cell Transplantation and Gene Therapy Approaches

Brief Description of Technology

Defined population predicts and quantitatively correlates with *in vivo* engraftment and multilineage potential.

BUSINESS OPPORTUNITY
Exclusive license
Sponsored research

TECHNOLOGY TYPE
Platform technology
Gene therapy
Cell therapy
Cell manufacturing
Therapeutic

STAGE OF DEVELOPMENT
Preclinical *in vivo*

PATENT INFORMATION
Patent pending

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Technology Overview

Hematopoietic stem cells (HSC) are the preferred target population for *ex vivo* gene therapy with applications ranging from rare monogenetic diseases to HIV. Currently, HSCs are isolated by the marker CD34. However, use of this population has severe limitations. Fred Hutch researchers have identified a unique combination of genetic markers that define a small subset within the CD34-expressing population, which represents HSCs with self-renewing capacity, multilineage potential, long-term engraftment capability and is conserved between human and nonhuman primates. This method to isolate, expand, and manipulate HSCs has dramatic potential to reduce the cost of goods for manufacturing of gene and cell based therapeutics and provides a quantitative measure of graft potency.

Applications

- Selection of HSCs for autologous transplantation
- Isolation of HSCs for engineering gene and cell based therapies

Advantages

- Predictability that isolated cell population will engraft *in vivo*
- Self-renewal and multi-lineage potential of HSCs
- 20 fold reduced vector requirements for gene manipulation

Market Overview

Over 40 million people globally are suffering from hematopoietic diseases and HSC transplantation (HSCT) is an attractive approach for curative treatment. From 2009 to 2013, there were 88,063 HSC transplants performed in the US. While HSCTs are on the rise, the use of gene therapy HSC strategies is limited due to barriers in manufacturing associated with the significant cost of clinical grade vectors and reagents. These identified HSC populations would allow for more predictable results of HSCT and also reduce vector and reagent requirements given the fewer cell numbers required for predictable long-term multi-lineage engraftment.