



CELL ENGINEERING

Methods to Target HSCs for Ablation or Expansion

Brief Description of Technology

Luteinizing hormone receptor (LHR) binding agents and luteinizing hormone (LH) agonists to target and expand HSCs.

BUSINESS OPPORTUNITY

Exclusive license
 Non-exclusive license
 Sponsored research
 Start-up

TECHNOLOGY TYPE

Cell therapy
 HSC therapy
 Research tool

STAGE OF DEVELOPMENT

Preclinical *in vitro* and *in vivo*

PATENT INFORMATION

Patent pending

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Technology Overview

Hematopoietic stem cell transplant (HCT) has been curative for many cancer malignancies and monogenetic diseases. However, current clinical limitations include small number of true HSCs in the transplant graft and the need for genotoxic myeloablative conditioning regimens. Teams at Fred Hutch and Memorial Sloan Kettering have discovered a novel role of LH in HSC biology and have demonstrated expansion of primitive HSC populations in the presence of LH. Furthermore, the teams are developing novel therapeutic agents to target the LHR receptor to specifically ablate HSCs as a non-myeloablative conditioning regimen prior to HCT.

Applications

- *Ex vivo* expansion of HSCs for cell and gene therapy
- Selective ablation of HSCs *in vivo* as a conditioning regimen [in progress]
- Selective isolation of human and mouse HSCs from mixed tissue suspensions
- Therapeutic approach to promote hematopoietic recovery after radiation injury

Advantages

- High LHR expression on the most primitive HSC populations allows for selective isolation of this cell population from mouse and man
- Selective ablation of HSCs would allow for non-genotoxic conditioning in vulnerable patient populations
- Targeting this pathway can also promote survival following a hematopoietic lethal dose of irradiation

Market Overview

Over 40 million people globally are suffering from hematopoietic diseases and HCT offers an attractive approach for curative treatment. From 2010 to 2014, there were 92,784 HSC transplants performed in the US. The conditioning regimen is a critical step in HCT and depending on method can have variable intensity, toxicity, and dependence upon a graft-versus-tumor effect. Thus opportunities exist for novel methods to improve conditioning methods.