Ex Vivo Maintenance and Expansion Of Hematopoietic Stem Cells

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BACKGROUND

Hematopoietic stem cell (HSC) transplants are used to treat patients with a broad spectrum of hematological malignancies, immune disorders and genetic blood diseases. Unfortunately, even after decades of use and research, there is a significant shortage of histocompatible HSCs available for transplants. Transplanting larger numbers of HSCs increases the likelihood and speed of successful engraftment, which can reduce the risk of complications such as anemia and infection, and more effectively treat underlying disease. The inability to efficiently maintain adult HSCs *ex vivo* is also a significant barrier for the wider development and implementation of gene therapies for diverse blood diseases and a major obstacle for engineering HSC derived cellular products for immunotherapy. One approach to overcome this challenge is to develop a means to maintain and expand HSCs in culture. Unfortunately, there is no well-defined reproducible means to maintain or expand HSCs. Even short culture times in optimized conditions are deleterious to HSCs. *Ex vivo* HSC maintenance and expansion could significantly enhance their clinical utility in a wide range of human diseases, providing a new platform for testing drugs, enabling more efficient gene editing within stem cells, and developing into a widely-used tool for the research community.

TECHNOLOGY DESCRIPTION

Researchers at UC San Diego have discovered and pioneered a method to maintain and expand adult HSCs *ex vivo*. This method utilizes interventions that modulate protein biogenesis and homeostasis pathways that are essential for stem cell self-renewal, but that become dysregulated in culture. Experiments have demonstrated that the method fully supports robust maintenance and expansion of HSCs in culture for at least 10 days. Cultured HSCs completely maintain their regenerative capacity as they are fully competent to give long-term multilineage reconstitution upon serial transplantation.

APPLICATIONS

The invention enables the prolonged maintenance and expansion of HSCs in culture. This could support the expansion or treatment of adult HSCs prior to transplantation. It could also provide a safe and effective platform to test drugs on adult HSCs. The invention could also provide a platform that enables efficient genetic modification/editing of adult HSCs, which could facilitate the treatment of genetic blood disorders and enhance the generation of engineered cell and immune therapy products.

ADVANTAGES

The discovery offers a unique method and novel approach that enables *ex vivo* expansion of adult HSCs. It also allows for long-term maintenance of HSCs culture without a loss of their regenerative activity.

STATE OF DEVELOPMENT

The invention is in the experimental data stage.

INTELLECTUAL PROPERTY INFO

This technology is patent pending and available for licensing and/or research sponsorship.

PATENT STATUS

Country	Type	Number	Dated	Case
Patent Cooperation Treaty	Published Application	2019055817	03/21/2019	2017-337

Additional Patent Pending

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OTHER INFORMATION

KEYWORDS

stem cell, stem cell transplant, therapeutics, hematopoietic stem cells, hsc, cell therapy, immunotherapy, proteostasis, leukemia, lymphoma, hematological malignancy, bone marrow transplant

CATEGORIZED AS

- Medical
  - Gene Therapy
  - Stem Cell

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