

## Method For Combined Conditioning And Chemoselection In A Single Cycle Of Hematopoietic Stem Cell Transplantation

Tech ID: 22098 / UC Case 2011-478-0

### SUMMARY

Researchers at UCLA have developed a highly-efficient technique to engraft and select hematopoietic stem cells directly inside a patient. The invention will improve the fidelity of stem cell transplantations and reduce toxic side effects on patients.

### BACKGROUND

Hematopoietic stem cell transplantation (HSCT) is a mainstay of treatment for many hereditary disorders and lymphatic and blood cancers. However, HSCT regimens are maligned with poor transplantation efficiency and patient complications. For instance, the toxic side effects associated with chemotherapy or radiation-mediated pre-conditioning can compromise patient survival. In addition, the poor rate of transplanted cell engraftment and insufficient supplies of donor cells has limited the use and efficacy of HSCT. Therefore, there is an urgent need to improve the efficiency of engraftment and lower the toxicity of preconditioning regimens. Advancing these phases of HSCT will improve patient outcomes by reducing risks from preconditioning, overall durations of treatment, and costs from extended hospitalization and multiple transplantations.

### INNOVATION

UCLA scientists have developed a unique and novel chemoselection technique that achieves high efficiency of engraftment and long-term reconstitution. By utilizing genetically modified donor cells that are resistant to preconditioning, myeloablative compounds, researchers have fused the preconditioning and the transplant chemoselection phases of treatment into a single therapeutic cycle. This allows more rapid engraftment, a reduced preconditioning phase, and a reduction in the concentration of drugs required for both preconditioning and chemoselection.

### APPLICATIONS

- ▶ Hematopoietic stem cell transplantation.
- ▶ Applications to future therapies involving transplantation of human stem cells

### ADVANTAGES

- ▶ Highly efficient engraftment and reconstitution
  - ▶ Replacement of >95% of recipient bone marrow
- ▶ Reduced time to engraftment.
- ▶ Preconditioning does not require irradiation.
- ▶ The dose of preconditioning and chemoselection drugs in the invention is 3-fold lower than that required for other HSCT regimens.

### STATE OF DEVELOPMENT

The novel HSCT technique has been tested and validated in murine models. The kinetics and composition of bone marrow reconstitution has also been documented. Methods to genetically modify human cells for HSCT are being optimized for translational studies.

### PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	11,607,427	03/21/2023	2011-478
United States Of America	Issued Patent	10,231,998	03/19/2019	2011-478

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### OTHER INFORMATION

#### KEYWORDS

Hematology, Hematopoietic stem cell transplantation, chemoselection, bone marrow, stem cells

#### CATEGORIZED AS

- ▶ **Medical**
  - ▶ Disease: Blood and Lymphatic System
  - ▶ Stem Cell
  - ▶ Therapeutics

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2011-478-0

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