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## Targeted Immunotherapy-Based Blood Stem Cell Transplantation

### Grant Award Details

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Targeted Immunotherapy-Based Blood Stem Cell Transplantation

**Grant Type:** Quest - Discovery Stage Research Projects

**Grant Number:** DISC2-13400

**Project Objective:** The objective of this project is to develop an engineered antibody construct that targets and recruits immune cells to kill diseased blood stem cells, including leukemia stem cells, so that healthy stem cells can replace the diseased ones.

**Investigator:**

<b>Name:</b>	Judith Shizuru
<b>Institution:</b>	Stanford University
<b>Type:</b>	PI

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**Disease Focus:** Blood Disorders

**Human Stem Cell Use:** Other

**Award Value:** \$1,341,910

**Status:** Pre-Active

### Grant Application Details

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**Application Title:** Targeted Immunotherapy-Based Blood Stem Cell Transplantation

**Public Abstract:****Research Objective**

An engineered antibody construct that targets and recruits immune cells to kill diseased blood stem cells, including leukemia stem cells, so that healthy stem cells can replace the diseased ones

**Impact**

An antibody that can direct immune cells to kill diseased stem cells would make stem cell transplant safer, more accessible and effective for the treatment of many life-threatening blood disorders

**Major Proposed Activities**

- Design and produce antibody constructs that can direct immune cells to bind to and kill normal and malignant blood stem cells
- Test the candidate antibody constructs in cell culture assays to determine rank which constructs are the most potency at killing target cells
- Select the top candidate constructs for testing in mice that have been engrafted with malignant human stem cells
- Perform scale up production of the top candidates
- Test administration of top constructs in mice that have been engrafted with malignant human stem cells to determine if the treatment results in permanent eradication of the diseased cells
- Treat mice engrafted with malignant human stem cells with combined construct + transplants of normal human stem cells to determine if diseased cells are eradicated and replaced by healthy stem cells

**Statement of Benefit to California:**

Almost 10,000 Californians with blood diseases received a blood stem cell transplant (SCT) in the past 10 years. Although the SCT can be curative, many suffer serious and life-threatening side effects due in part to toxic chemotherapy and radiation used in the SCT. Thus, transplant is costly and unavailable to thousands of underserved Californians with blood diseases. Replacing toxic chemoradiation with a non-toxic antibody can minimize the hardships of SCT and open it to the underserved.

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