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## Human Embryonic Stem Cell-Derived Natural Killer Cells for Cancer Treatment

### Grant Award Details

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Human Embryonic Stem Cell-Derived Natural Killer Cells for Cancer Treatment

**Grant Type:** Therapeutic Translational Research Projects

**Grant Number:** TRAN1-10587

**Project Objective:** To enable an FDA pre-IND meeting for an allogeneic "off-the-shelf" human embryonic stem cell (hESC)-derived natural killer (NK) cell immunotherapy for the treatment of acute myelogenous leukemia (AML)

**Investigator:**

<b>Name:</b>	Dan Kaufman
<b>Institution:</b>	University of California, San Diego
<b>Type:</b>	PI

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**Disease Focus:** Acute Myeloid Leukemia, Blood Cancer, Cancer

**Human Stem Cell Use:** Embryonic Stem Cell

**Award Value:** \$4,698,821

**Status:** Active

### Grant Application Details

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**Application Title:** Human Embryonic Stem Cell-Derived Natural Killer Cells for Cancer Treatment

**Public Abstract:****Translational Candidate**

Human embryonic stem cell (hESC)-derived natural killer (NK) cells to target relapsed/refractory Acute Myelogenous Leukemia (AML)

**Area of Impact**

hESC-derived NK cells provide a novel and potent approach to treat relapsed or refractory AML that is resistant to current chemotherapy options.

**Mechanism of Action**

hESC-derived NK cells provide a standardized, homogeneous, off-the-shelf cellular immunotherapy product that will be used as an allogeneic adoptive transfer treatment for patients with AML who have either never achieved remission with standard induction therapy, or who relapse after previous chemotherapy. hESC-derived NK cells kill tumor cells by several mechanisms: direct cytotoxicity, antibody-dependent cell-mediated cytotoxicity, induction of apoptosis and production of cytokines.

**Unmet Medical Need**

Over 10,000 in the US die each year from AML, with 5 year survival <30%. Allogeneic NK cells are known to destroy AML cells in patients who have failed chemotherapy. hESC-derived NK cells will provide the first standardized, "off-the-shelf" cellular immunotherapy to treat this deadly disease.

**Project Objective**

The objective is to have an FDA Pre-IND meeting

**Major Proposed Activities**

- We will use the GMP hESC line ESI-017 to produce a Master Cell Bank and Working Cell Bank of NK cells using defined clinical-scale cell methods.
- We will demonstrate ESI-017 hESC-derived NK cells kill AML tumor cells 1) in vitro, and 2) in vivo using NSG mouse xenograft models.
- We will assess safety of ESI-017 hESC-derived NK cells using NSG mouse model and in vitro assays to test tumorigenicity and lack of off-target killing

**Statement of Benefit to California:**

Over a thousand Californians are diagnosed with Acute Myeloid Leukemia (AML) each year, and five year survival in California is less than 30%. New treatment options are desperately needed for patients who fail standard chemotherapy. We will produce a standardized, off-the-shelf immunotherapy cell product that can induce remissions and lead to cure of AML. These studies with hESC-derived NK cells will allow Californians to be at the forefront of this cellular immunotherapy approach to treat AML

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