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**Genetic Modification of Stem Cells and T cells to Activate the Immune System to Target Solid Tumors**

**Grant Award Details**

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Genetic Modification of Stem Cells and T cells to Activate the Immune System to Target Solid Tumors

**Grant Type:** Clinical Trial Stage Projects

**Grant Number:** CLIN2-11380

**Project Objective:** Complete a Phase 1 clinical trial for advanced solid cancer (Synovial Carcinoma) with NY-ESO-1 T cell receptor engineered peripheral blood mononuclear cells (PBMC) and peripheral blood stem cells (PBSC).

**Investigator:**

<b>Name:</b>	Theodore Nowicki
<b>Institution:</b>	University of California, Los Angeles
<b>Type:</b>	PI

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**Disease Focus:** Cancer, Sarcoma, Solid Tumors

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$4,693,839

**Status:** Active

**Grant Application Details**

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**Application Title:** Genetic Modification of Stem Cells and T cells to Activate the Immune System to Target Solid Tumors

**Public Abstract:****Therapeutic Candidate or Device**

Autologous Peripheral Blood Stem Cells expressing the NY-ESO-1 TCR and a suicide/reporter gene combined with T cells expressing the same TCR

**Indication**

Locally advanced (unresectable stage IIIc) or metastatic malignancies (stage IV) that are HLA A2.1 +, NY-ESO-1 +, solid tumors, including sarcomas

**Therapeutic Mechanism**

The administration of TCR transduced mature lymphocytes will expand in vivo and provide a first wave of transient antitumor activity. This will provide a bridge until the genetically modified CD34+ cells expressing a transgenic NY-ESO-1 TCR give rise to T cells recognizing the NY-ESO-1 antigen presented by HLA-A2\*0201 in NY-ESO-1 positive malignant cells generating a renewable source of TCR transduced cells for sustained antitumor activity.

**Unmet Medical Need**

The rarity of sarcomas limits funding and available treatments. This trial will constitute one of the very few options for patients with relapsed or recurrent sarcoma, who have a high prevalence of NY-ESO-1 tumor expression, as well as other types of solid tumors with high NY-ESO-1 expression.

**Project Objective**

Phase 1 trial completed

**Major Proposed Activities**

- Vectors production and assess feasibility of cell product manufacturing
- Assess clinical safety, T cell persistence and anti-tumor response of the combination of the cell products administered
- Assess biodistribution of the modified stem cells and progeny

**Statement of Benefit to California:**

The clinical success would foster the modification of our current techniques for cell therapy manufacture to be adapted to a larger scale manufacturing procedure compatible with commercialization. This would broaden the impact of this stem cell-based research for patients in California and the US.

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