Ex Vivo Gene Engineering of Blood Stem Cells for Enhanced Chemotherapy Efficacy in Glioblastoma Patients

Grant Award Details

Ex Vivo Gene Engineering of Blood Stem Cells for Enhanced Chemotherapy Efficacy in Glioblastoma Patients

Grant Type: Late Stage Preclinical Projects

Grant Number: CLIN1-10967

Project Objective: Engineer an Ex Vivo Gene from Blood Stem Cells for Enhanced Chemotherapy Efficacy in Glioblastoma Patients

Investigator:

Name:	John Zaia
Institution:	City of Hope, Beckman Research Institute
Type:	PI

Disease Focus: Brain Cancer, Cancer, Solid Tumors

Human Stem Cell Use: Adult Stem Cell

Award Value: $3,684,259

Status: Active

Grant Application Details

Application Title: Ex Vivo Gene Engineering of Blood Stem Cells for Enhanced Chemotherapy Efficacy in Glioblastoma Patients

Public Abstract:

Therapeutic Candidate or Device

Blood stem cells will be genetically engineered to protect them from chemotherapy in glioblastoma patients, producing better patient survival.

Indication

Patients with newly diagnosed glioblastoma (GBM) multiforme, or any grade IV newly diagnosed glioma, will be eligible to receive this therapy.

Therapeutic Mechanism

Chemotherapy is the first-line treatment for GBM, but it is associated with toxic side effects in the blood, limiting the amount of drug a patient can tolerate. Our therapeutic candidate, genetically protected blood stem cells, will decrease the side-effects of the chemotherapy, allowing higher doses of this chemotherapy to be given. This should increase the amount of tumor killing , produce better quality of life, and improved overall survival in these GBM patients.

Unmet Medical Need

There is no cure for glioblastoma. Patient's survival remains ~15 months and treatment involves a type of chemotherapy limited by its toxicity. Our strategy will improve the quality of life and overall survival by reducing these side-effects and allowing more anti-tumor treatment to be given.

Project Objective

IND filing and initiation of Phase 1 trial sites

Major Proposed Activities

- Optimize the manufacturing of the therapeutic product. This phase of the project will lead to 3 production runs under clinical trial conditions.
- Characterize the efficacy and safety profiles of the therapeutic product. The product will be tested for safety at the collaborating site FHCRC.
- Prepare the IND application and multi-site clinical trial initiation. This will describe the manufacturing, safety, and clinical trial details.

Statement of Benefit to California:

GBM is the most common and aggressive primary brain tumor in adults and ~1500 new cases are diagnosed per year in CA. The 5 year relative survival rate for GBM is 4.8 % in CA and has remained at this low level over the last two decades. In addition, the main treatment involves chemotherapy which is effective but limited by its toxicity. If a process can protect from such toxicity, Californians will greatly benefit from a significant improvement over the current first-line therapy of GBM.
