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**Phase 1 Clinical Trial of Autologous GD2 Chimeric Antigen Receptor T Cells for Diffuse Intrinsic Pontine Gliomas and Spinal Diffuse Midline Glioma**

**Grant Award Details**

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Phase 1 Clinical Trial of Autologous GD2 Chimeric Antigen Receptor T Cells for Diffuse Intrinsic Pontine Gliomas and Spinal Diffuse Midline Glioma

**Grant Type:** Clinical Trial Stage Projects

**Grant Number:** CLIN2-12595

**Project Objective:** Complete a Phase 1 clinical trial of autologous GD2 CAR-T cells for the treatment of Diffuse Intrinsic Pontine Gliomas (DIPG) or Spinal Diffuse Midline Gliomas (DMG)

**Investigator:**

<b>Name:</b>	Crystal Mackall
<b>Institution:</b>	Stanford University
<b>Type:</b>	PI

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

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

**Award Value:** \$11,998,310

**Status:** Active

**Grant Application Details**

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**Application Title:** Phase 1 Clinical Trial of Autologous GD2 Chimeric Antigen Receptor T Cells for Diffuse Intrinsic Pontine Gliomas and Spinal Diffuse Midline Glioma

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

Autologous T cells genetically engineered to express a Chimeric Antigen Receptor targeting GD2 (GD2-CART)

**Indication**

Brain tumors in children and young adults: Diffuse Intrinsic Pontine Gliomas (DIPG) and Spinal Diffuse Midline Glioma (DMG)

**Therapeutic Mechanism**

Progenitor GD2-CART cells will recognize DIPG/DMG cancer cells expressing GD2, become activated, divide, and kill the cancer cells.

**Unmet Medical Need**

DIPG, the leading cause of childhood brain tumor death, is uniformly fatal. Many clinical trials have explored the use of various therapeutic agents for DIPG. However, no improvement in overall survival has been demonstrated to date. Thus, there is an urgent need for novel effective therapies.

**Project Objective**

Phase 1 trial completed

**Major Proposed Activities**

- Determine recommended Phase 2 dose of therapeutic for patients with DIPG and Spinal DMG
- Assess toxicity of GD2CAR T cells
- Assess clinical activity of GD2CAR T cells in children and young adults with DIPG and Spinal DMG.

**Statement of Benefit to California:**

Brain tumors are the leading cause of cancer related death in children. Among these, DIPG and DMGs are the most aggressive and are universally fatal with current standard therapies, with median overall survival of 11 months for DIPG. We propose to conduct a Phase I clinical trial of novel GD2 targeting CAR T cells, which have shown impressive antitumor activity in preclinical studies. If successful, this therapy would transform the landscape for this universally lethal pediatric brain tumor.

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