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**Treatment of sickle cell disease by induction of mixed chimerism and immune tolerance using CD4+ T-depleted haploidentical blood stem cell transplant**

**Grant Award Details**

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Treatment of sickle cell disease by induction of mixed chimerism and immune tolerance using CD4+ T-depleted haploidentical blood stem cell transplant

**Grant Type:** Clinical Trial Stage Projects

**Grant Number:** CLIN2-10847

**Project Objective:** Complete the Phase 1 clinical trial

**Investigator:**

<b>Name:</b>	Joseph Rosenthal
<b>Institution:</b>	City of Hope, Beckman Research Institute
<b>Type:</b>	PI

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**Disease Focus:** Blood Disorders, Sickle Cell Disease

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

**Award Value:** \$5,742,180

**Status:** Active

**Grant Application Details**

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**Application Title:** Treatment of sickle cell disease by induction of mixed chimerism and immune tolerance using CD4+ T-depleted haploidentical blood stem cell transplant

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

Haploidentical (half-match) T cell depleted blood stem cell transplant with a low-toxic conditioning regimen

**Indication**

Adult patients with severe sickle cell disease who are excluded from the potentially curative current standard stem cell transplant.

**Therapeutic Mechanism**

The proposed therapy is intended to achieve mixed chimerism and immune tolerance. Mixed chimerism is when a combination of donor and host blood cells co-exist in the transplanted host. The right mix of donor to host blood cells can reverse sickle cell disease. Immune tolerance will prevent rejection of the donor blood stem cell graft and allow patients to be free of sickle cell disease for a long time.

**Unmet Medical Need**

This proposal will allow more people with severe sickle cell disease to have a potentially curative stem cell transplant. Our method will allow patients to receive less-toxic conditioning drugs before the transplant, and to get stem cells from half-match donors .

**Project Objective**

Complete the Phase 1 clinical trial

**Major Proposed Activities**

- Manufacture a half-match T-cell-depleted blood stem cell donor product
- Conduct a clinical trial with severe sickle cell disease patients
- Assess safety and ability to induce mixed chimerism

**Statement of Benefit to California:**

About 7000 people in California are living with severe sickle cell disease (SCD). Currently, severe SCD patients need lifelong supportive care and extensive health care management, including social and community services. We will develop a new product with the potential to cure severe SCD. Curing severe SCD will improve the health and well-being of California citizens who suffer from this disease, eliminate their lifelong dependence on supportive care, and significantly reduce health care costs.

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