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:

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<tr>
<th>Name</th>
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<tr>
<td>Institution</td>
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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

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.