



Current Status: Comprehensive Sickle Cell Centers Clinical Trials Consortium Multi-Center Collaborative Studies



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Purpose

Collaborative efforts among multiple medical centers are necessary to address important clinical questions. The NHLBI Comprehensive Sickle Cell Centers Clinical Trials Consortium (CSCC CTC) consists of 10 Centers (each with multiple clinical sites) and a Statistics and Data Management Center (SDMC) whose primary objectives are to carry out research focused on improving our understanding of sickle cell disease (SCD) and testing new therapeutic approaches. As Year 03 of the current funding cycle has begun (April 1, 2005), four multi-center collaborative studies are active, three of which are recruiting patients from the 10 Centers. Five additional studies are in development.

SDMC Roles

- Facilitate/assist in protocol development
- Coordinate preparation of study materials
- Coordinate central and on-site training for clinical site staff
- Monitor recruitment and accrual of study participants
- Oversee data collection, data management, quality control and analyses
- Collaborate in the preparation of scientific reports and publications
- Serve as liaison between the investigator and the Protocol Review Committee, the Data Safety and Monitoring Board, and study sites
- Prepare technical and administrative reports for PRC, DSMB, and other groups

Participating Centers and Directors

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Bronx Comprehensive Sickle Cell Center

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Comprehensive Sickle Cell Center

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Studies in Progress

Title: Arginine Supplementation in Sickle Cell Anemia: Physiological and Prophylactic Effects

Objective: Phase II study to assess the physiological effects (both deleterious and beneficial) of the administration of oral arginine, a dietary supplement, in patients with SCD

Population: SS patients, 48 pediatric (> 5 years of age) and 48 adult patients

Sites: 17 clinical sites from 8 Centers

First subject enrolled: June 8, 2004

Projected end date: December 2007

Progress to date: 48 enrolled subjects

Title: Neuropsychological Dysfunction and Neuroimaging Abnormalities in Intact Adult Patients with Sickle Cell Disease

Objective: Part I – Determine the extent of neurocognitive dysfunction in neurologically asymptomatic adult patients with SCD
Part II – Pilot study to assess the effect of transfusion on a subset of Phase I subjects with decreased neurocognitive function

Population: Part I – SS and Sβ⁰ patients, 120 adult and 36 community-matched controls (Hb AA);
Part II – SS and Sβ⁰ patients, a subset of 30 patients from Part I

Sites: 10 clinical sites from 7 Centers

First subject enrolled: December 1, 2004

Projected end date: December 2007

Progress to date: 10 enrolled subjects;
1 enrolled community-matched control

Title: Collaborative Data Project

Objective: To establish a comprehensive database of clinical and diagnostic data on sickle cell patients from all participating Centers

Population: All SCD genotypes, 3,400 pediatric and 1,200 adult patients

Sites: 24 clinical sites from 10 Centers

First subject enrolled: March 8, 2005

Projected end date: March 2008. The continuation of this collection effort beyond the funding cycle will be a priority with a goal of 10,000 enrolled patients.

Progress to date: 5 enrolled subjects

Comment: Expansion to include a DNA sample analysis storage repository is being planned.

Title: Epidemiology of Priapism

Objective: Conduct a large-scale survey to enumerate the cross-sectional prevalence, demographics, and common clinical characteristics of priapism in males with SCD among the CSCC population

Population: SS and Sβ⁰ patients, 1,400 males > 5 years of age; SC and Sβ⁺ patients, 250 males > 15 years of age

Sites: 20 clinical sites from 8 Centers have agreed to participate

Projected start date: May 15, 2005

Projected end date: December 2006

Progress to date: Centralized interviewer training is expected to occur in mid to late May.

Studies in Development

Title: Effectiveness of Hydroxyurea and Magnesium Pidolate Alone and in Combinations in Hemoglobin SC Disease

Objective: Phase II study to compare the effectiveness of hydroxyurea alone, magnesium pidolate (Mg) alone, and hydroxyurea + Mg in combination, compared to placebo in reducing the density of Hb SC erythrocytes

Population: SC patients, 160 patients > 5 years of age

Projected start date: September 2005

Project duration: 36 months

Title: An Extended Phase II Study of Decitabine in Subjects with High-Risk Sickle Cell Disease

Objective: Phase I/II study to determine the optimal dose and frequency per week of decitabine administration to produce HbF elevations of > 30% without toxicity

Population: All SCD genotypes, 32 adult patients

Projected start date: November 2005

Project duration: 24 months

Title: Randomized Trial of Oral Dexamethasone for Acute Chest Syndrome

Objective: Phase III study to determine whether administration of the steroid dexamethasone decreases the duration of hospitalization for individuals who have ACS

Population: All SCD genotypes, 110 pediatric (> 5 years of age) and 110 adult patients

Projected start date: December 2005

Project duration: 24 months

Title: Priapism II

Objective: To evaluate the effectiveness of nightly oral pseudoephedrine in reducing the number and duration of recurrent priapism events

Population: All SCD genotypes, 60 male patients

Projected start date: March 2006

Project duration: 24 months

Title: Headache Epidemiology and Prophylaxis Trial

Objective: Phase III study to determine the effects of antidepressants on the frequency and severity of migraine headaches in the sickle cell population

Population: All SCD genotypes, 600 patients > 7 years of age in part A; 200 patients 7-17 years of age in part B; 160 patients 18-50 years of age in part C

Projected start date: April 2006

Project duration: 12 months