



# Comprehensive Sickle Cell Centers Collaborative Data Project: Results From Year 1



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## Introduction

Collaborative efforts among multiple clinical centers are necessary to address important research questions. The NHLBI Comprehensive Sickle Cell Centers (CSCC) 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 to treatment. The purpose of this report is to describe the current status of the Collaborative Data (C-Data) project.

## Specific Aims

1. Provide a complete list and general overview of the centers' patient population.
2. Provide an information base for designing collaborative CSCC studies, especially sizes of subgroups of potential patients with specific characteristics, such as headache, Acute Chest Syndrome, etc. who meet specific inclusion/exclusion criteria.
3. Support identification of specific individuals eligible for observational as well as clinical studies, including treatment-specific HRQoL and outcome studies.
4. Provide basic HRQoL data on a large population of adults and children with SCD for comparison with population norms.
5. Provide the opportunity for exploration of relationships between patient characteristics and various events, and allow measurement and reporting of frequencies and patterns of specific sequelae, healthcare utilization, and economic burden associated with SCD and its complications.

## Study Overview

- At enrollment, the following are completed:
  - Informed Consent
  - Enrollment Form
  - Detailed Medical History Form (Part I)
  - Brief Medical History Interview (Part II)
  - Health-Related Quality of Life (HRQoL) and Patient Satisfaction Questionnaires
- Additional clinical and interview data will be obtained at annual or semiannual intervals.

## Data Management

- A remote, internet-based electronic data capture (EDC) system is used to capture study data.
- EDC is accessible via the CSCC website and requires user ID/password privileges.
- Site personnel log in to the system, review and correct previously entered data, or key additional data.
- Data are converted to analysis datasets using SAS (Version 8.0).

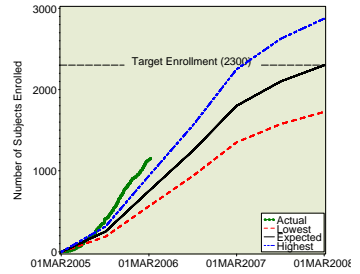
## Year 1 Summary

- 17 sites from 8 centers have received local IRB approval, 14 are active.
- Actual enrollment has exceeded projected enrollment to date among adult (377) and pediatric (777) subjects.
- Completed data (submission of Medical History Forms Part I and II) have been obtained for 65% of enrolled subjects. The delay in submission is due to the time required to collect the data; additionally, some forms require Quality Control review by the Principal Investigator prior to data entry.
- The most commonly reported medical condition among both adult and pediatric subjects was pain crisis, followed by acute chest syndrome.

### Participating Centers

Boston Comprehensive Sickle Cell Center	Bronx Comprehensive Sickle Cell Center
Children's Hospital of Philadelphia Comprehensive Sickle Cell Center	Cincinnati Comprehensive Sickle Cell Center
Duke-UNC Comprehensive Sickle Cell Center	Marian Anderson Sickle Cell Anemia Care and Research Center
Northern California Comprehensive Sickle Cell Center	St. Jude Children's Research Hospital Comprehensive Sickle Cell Center
University of Southern California Comprehensive Sickle Cell Center	University of Texas Southwestern Comprehensive Sickle Cell Center

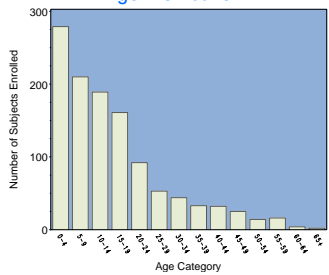
### Projected and Actual Enrollment



### Demographic Information

All Enrolled Subjects (N=1154)		
Age	Mean (SD)	15.7 (13.71)
	Min, Max	0, 68
Age Group - N (%)	< 18 yrs	777 (67)
	≥ 18 yrs	377 (33)
Gender - N (%)	Female	569 (49)
	Male	585 (51)
Diagnosis - N (%)	SS	737 (64)
	SC	255 (22)
	Sβ <sup>+</sup>	76 (7)
	Sβ <sup>0</sup>	56 (5)
	S Other	30 (3)
Race - N (%)	Black/Black Bircial	964 (84)
	Not Black/Black Bircial	49 (4)
Ethnicity - N (%)	Hispanic or Latino	35 (3)
	Not Hispanic or Latino	976 (85)

### Age Distribution



### History of Medical Conditions

Condition	< 18 yrs (N=586) N (%)	≥ 18 yrs (N=182) N (%)	Total (N=768) N (%) [1]
Subjects ever diagnosed with:			
Acute Chest Syndrome or Pneumonia	277 (47)	127 (70)	404 (53)
Aplastic Episode	86 (15)	17 (9)	103 (13)
Asthma/Reactive Airway disease	108 (18)	26 (14)	134 (17)
Avascular Necrosis, Hip(s)	11 (2)	35 (19)	46 (6)
Avascular Necrosis, Shoulder(s)	3 (<1)	18 (10)	21 (3)
Bacteremia/Sepsis	67 (11)	18 (10)	85 (11)
Central Nervous System Disease, Abnormal TCD	33 (6)	6 (3)	39 (5)
Central Nervous System Disease, Stroke, Ischemic	37 (6)	20 (11)	57 (7)
Dactylitis (Hand Foot Syndrome)	142 (24)	19 (10)	161 (21)
Gall Bladder Disease	45 (8)	79 (43)	124 (16)
Hypertension, chronic, requiring treatment	7 (1)	25 (14)	32 (4)
Leg Ulcer	2 (<1)	24 (13)	26 (3)
Liver Disease, Hepatitis, Viral	0	10 (5)	10 (1)
Liver Disease, Iron Overload	25 (4)	17 (9)	42 (5)
Lung Disease, Pulmonary Hypertension	2 (<1)	15 (8)	17 (2)
Osteomyelitis	10 (2)	10 (5)	20 (3)
Pain Crisis	353 (60)	168 (92)	521 (68)
Prisipism [2]	32 (10)	27 (30)	59 (15)
Renal Disease, Proteinuria	1 (<1)	9 (5)	10 (1)
Retinopathy	5 (<1)	21 (12)	26 (3)
Splenic Sequestration	123 (21)	17 (9)	140 (18)

### Summary of Patient Interview

	< 18 yrs (N=709) N (%)	≥ 18 yrs (N=307) N (%)	Total (N=1016) N (%) [1]
Current employment status:			
Full Time	-	54 (18)	-
Part Time	-	61 (20)	-
Not Employed	-	191 (62)	-
Current student status:			
Full Time	-	69 (22)	-
Part Time	-	27 (9)	-
Not a student	-	211 (69)	-
Not employed and Not a student	-	141 (46)	-
Health Insurance:			
Private	200 (28)	65 (21)	265 (26)
Medicare	48 (7)	72 (23)	120 (12)
Medicaid	408 (58)	156 (51)	564 (56)
None	6 (<1)	16 (5)	22 (2)
Other	98 (14)	88 (29)	186 (18)
Have had one or more unscheduled visits for pain during the past year	364 (51)	238 (78)	602 (59)
Currently uses:			
Tobacco	-	63 (21)	-
Alcohol	-	87 (28)	-
School or work days missed in the past year:			
0	303 (43)	146 (48)	449 (44)
1-5	135 (19)	44 (14)	179 (18)
6-10	96 (14)	26 (8)	122 (12)
>10	168 (24)	84 (27)	252 (25)
School or work days missed by primary caregiver(s) of pediatric subjects in the past year:			
0	325 (46)	-	-
1-5	152 (21)	-	-
6-10	93 (13)	-	-
>10	129 (18)	-	-

### History of Surgical Procedures

Procedure [1]	< 18 yrs (N=586) N (%) [1]	≥ 18 yrs (N=182) N (%)	Total (N=768) N (%) [2]
Subjects ever had:			
Tonsillectomy / Adenoidectomy	52 (9)	24 (13)	76 (10)
Splenectomy	57 (10)	26 (14)	83 (11)
Cholecystectomy	53 (9)	93 (51)	146 (19)
Hip Replacement	0	10 (5)	10 (1)
Laser Procedure of the Eye(s)	2 (<1)	15 (8)	17 (2)
Permanent Inwelding Line	24 (4)	25 (14)	49 (6)

[1] Procedures reported for fewer than 5% of adult and pediatric subjects are not listed and include Hip Core Procedure and Vitrectomy.  
 [2] The total population N is defined as the number of subjects who have completed the Medical History Form Part I.

## Next Steps

1. Quality of Life data will be analyzed and reported.
2. A supporting study will be added to obtain blood samples from participants to conduct genetic analyses and create a DNA sample repository.
3. Annual data collection will be modified to encompass specific clinical criteria for selected phenotypes in parallel with the genetic analyses.

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