

# Collaborative Data Project [C-DATA] of the Comprehensive Sickle Cell Centers Program

Zora R. Rogers<sup>1</sup>, Susan Lieff<sup>2\*</sup>, Marsha McMurray<sup>3\*</sup>, Carlton Dampier<sup>3\*</sup>, Winfred C. Wang<sup>4</sup>, Melanie Chelednik<sup>2\*</sup>, George R. Buchanan<sup>1</sup>, Kenneth I. Ataga<sup>5</sup>, Laura M. DeCastro<sup>6</sup>, Matthew M. Heeney<sup>7</sup>, Karen Kalinyak<sup>8</sup>, Kim Smith-Whitley<sup>9</sup>, Elliott Vichinsky<sup>10</sup>, and Ronald W. Helms<sup>2\*</sup>

<sup>1</sup>UT Southwestern, Dallas, TX; <sup>2</sup>RhoFED, Chapel Hill, NC; <sup>3</sup>St. Christopher's Hospital, Philadelphia, PA; <sup>4</sup>St. Jude Hospital, Memphis, TN; <sup>5</sup>UNC, Chapel Hill, NC;

<sup>6</sup>Duke, Durham, NC; <sup>7</sup>Children's Hospital, Boston, MA; <sup>8</sup>Children's Hospital, Cincinnati, OH; <sup>9</sup>Children's Hospital, Philadelphia, PA and <sup>10</sup>Children's Hospital, Oakland, CA.

## Project Overview

- Establish large, geographically diverse patient registry of persons regularly followed in the 10 NHLBI-funded Comprehensive Sickle Cell Centers
- Monitor participants' medical status over time
- Evaluate Quality of Life and ongoing medical events
- Serve as resource for clinical research: needs assessment, numbers of patients available, and clinical correlates for planned DNA based studies
- Opened to enrollment March 2005, now enrolling at 20 sites in 9 centers

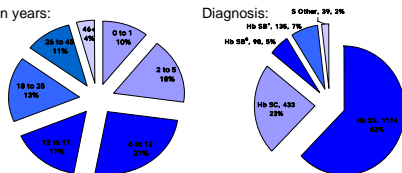
## Methods

- Formal written consent and HIPAA protection
- Enrollment data: patient interview and retrospective structured data abstraction from medical record
- Annual interview and semi-annual forms abstracted from medical record
- Common sickle cell events defined and specific minimum data required to confirm the diagnosis recorded

## Enrollment as of October 31, 2006

- Entry data on 1880 subjects: 1351 pediatric (< 18 years) and 529 adult
- 50% male

- Age in years:



- Diagnosis by Newborn Screen: 1325 or 70%

| Years followed in center: | ≤ 2 yrs | 3-5 yrs | 6-10 yrs | 11+ yrs |
|---------------------------|---------|---------|----------|---------|
|                           | 530     | 392     | 452      | 457     |
|                           | 28%     | 21%     | 24%      | 24%     |

## Clinical Events

### Lifetime Once or More

|                      | N total | Pediatric | Adult |
|----------------------|---------|-----------|-------|
| Painful Crisis       | 1338    | 64%       | 91%   |
| Chest Syndrome       | 1074    | 50%       | 74%   |
| CNS Events:          |         |           |       |
| - Ischemic Stroke    | 116     | 5%        | 9%    |
| - Hemorrhagic Stroke | 15      | <1%       | 2%    |
| - TIA                | 36      | 1%        | 3%    |
| Proteinuria          | 51      |           | 8%    |
| Renal Failure:       |         |           |       |
| - Acute              | 31      |           | 5%    |
| - Chronic            | 13      |           | 2%    |

### Surgery - Lifetime

|                       | N   | Age (%) |        | Genotype (%) |    |     |
|-----------------------|-----|---------|--------|--------------|----|-----|
|                       |     | Peds    | Adults | SS           | SC | S/β |
| Cholecystectomy       | 385 | 10      | 47     | 26           | 11 | 8   |
| Splenectomy           | 202 | 9       | 14     | 12           | 6  | 5   |
| Tonsillectomy         | 210 | 10      | 14     | 13           | 8  | 5   |
| Tunneled Central Line | 144 | 5       | 15     | 10           | 4  | 3   |

### Other Therapies

|                          |      |    |    |    |    |    |
|--------------------------|------|----|----|----|----|----|
| Transfusion:             |      |    |    |    |    |    |
| - In the last year       | 613  | 27 | 46 | 44 | 11 | 10 |
| - Prior to the last year | 1032 | 47 | 75 | 68 | 33 | 21 |
| Hydroxyurea              |      |    |    |    |    |    |
| - In the last year       | 453  | 18 | 39 | 32 | 8  | 8  |
| - Prior to the last year | 452  | 17 | 42 | 31 | 8  | 9  |

## Health Services Utilization

### Hospitalization in Prior 2 Years

|                        | N    | Age Group (%) |        | Genotype (%) |    |     |
|------------------------|------|---------------|--------|--------------|----|-----|
|                        |      | Peds          | Adults | SS           | SC | S/β |
| Total Hospitalizations | 4100 | 63            | 37     | 73           | 16 | 4   |
| For Pain Crisis        | 1843 | 50            | 50     | 72           | 17 | 4   |
| For Acute Chest        | 513  | 70            | 30     | 77           | 14 | 3   |
| For Fever/Infection    | 828  | 93            | 7      | 69           | 18 | 6   |
| Splenic Sequestration  | 90   | 100           | 0      | 77           | 9  | 6   |

### Hospitalizations per Person in Prior 2 Years

|                       |      |    |    |    |    |    |
|-----------------------|------|----|----|----|----|----|
| Admission 1 or more   | 1228 | 64 | 68 | 71 | 57 | 49 |
| For Pain Crisis       | 642  | 29 | 48 | 39 | 28 | 21 |
| For Acute Chest       | 358  | 19 | 19 | 22 | 14 | 9  |
| For Fever/Infection   | 465  | 31 | 8  | 27 | 22 | 17 |
| Splenic Sequestration | 56   | 4  |    | 5  | 2  | 2  |

### Other Events per Person in Prior Year

|                         |      |    |    |    |   |   |
|-------------------------|------|----|----|----|---|---|
| MRI brain               | 261  | 15 | 10 | 18 | 4 | 9 |
| Prophylactic Penicillin | 1006 | 65 | 24 |    |   |   |
| Other research trial    | 422  | 20 | 29 |    |   |   |

### Hospitalizations per Person in Prior Year

|           | Median | Mean | Range |
|-----------|--------|------|-------|
| Pediatric | 1      | 1.9  | 0-28  |
| Adult     | 1      | 2.9  | 0-60  |
| Hb SS     | 1      | 2.5  | 0-33  |
| Hb SC     | 1      | 1.6  | 0-60  |
| Hb S/β    | 0      | 1.3  | 0-9   |

## Screening

|                              | N   | Pediatric | Adult |       |       |       |     |
|------------------------------|-----|-----------|-------|-------|-------|-------|-----|
| Pulmonary Hypertension       |     |           |       |       |       |       |     |
| - Echo in past 3 years       | 431 | 14%       | 45%   |       |       |       |     |
| - Abnormal Echo past 3 years | 169 | 5%        | 20%   |       |       |       |     |
| - Dx. Pulmonary Hypertension | 46  | <1%       | 8%    |       |       |       |     |
| TCD Screening in past year   | 297 | 22%       | 1%    |       |       |       |     |
| - Age in years               |     |           |       |       |       |       |     |
|                              | 0-1 | 2-5       | 6-12  | 13-17 | 18-25 | 26-45 | 46+ |
|                              | 1%  | 28%       | 24%   | 15%   | 1%    | 1%    | 0%  |

### Hydroxyurea Use by Age Group

|                    | 0-1 | 2-5 | 6-12 | 13-17 | 18-25 | 26-45 | 46+ |
|--------------------|-----|-----|------|-------|-------|-------|-----|
| In the last year   | 2%  | 9%  | 24%  | 31%   | 39%   | 43%   | 29% |
| Prior to last year | 1%  | 5%  | 31%  | 29%   | 41%   | 50%   | 25% |

## Conclusions

- The C-DATA Project is a feasible method to collect clinical data on persons with sickle cell disease
- Sickle Cell Anemia (SS) patients experience the majority of the complications but significant morbidity occurs in other genotypes
- Screening tests (TCD, cardiac echo) are not uniformly obtained
- This database will be an invaluable tool for assessment of adherence to recommendations for care, health services planning, and outcomes assessment
- The C-DATA Project data will be useful for planning multi-center research studies, identifying specific individuals potentially eligible for participation in intervention trials, and providing clinical data to support DNA based genotype-phenotype studies