Supporting Statement
Sickle Cell Disease Treatment Demonstration Program

B. Collection of Information Employing Statistical Methods

I. Respondent Universe and Sampling Methods

The respondent universe for this study includes all the individuals with SCD and their caretakers enrolled in the SCDTDP. The approximate total number of the respondents is 400 individuals or 100 participants per Network. The small size of the universe does not warrant the imposition of a sampling technique. Clients were enrolled into the SCDTDP through multiple processes such as referrals, identifying individuals without care, and self selection.

2. Information Collection Procedures

Data will be collected at two points in time: 1) at baseline when the patients and caregivers are enrolled into the SCDTDP and 2) at 12 months following enrolment. The study will enrol participants on a rolling basis such that new patients will be added to the study over a specified period. At the time of enrolment, SCDTDP participants will be informed about the data collection as part of the informed consent process. Four different instruments have been selected/developed to collect the information for this study and are summarized in **Exhibit 2.1**. These include the:

- **SF-36 ®–** an instrument developed for the Medical Outcomes Study in the 1980's and which has been widely adopted for the measurement of functional health and well being in many other subsequent studies. The validity of SF-36 scales based on psychometric studies has been supported in results from clinical trials comparing scores for patients before and after treatment. The reliability of the eight scales and two summary measures has been estimated using both internal consistency and test-retest methods, and generally

have exceeded the minimum standard of 0.70. This instrument is designed to be self-administered.

- **PedsQL ® - The Pediatric Quality of Life Inventory** is a set of instruments developed by James W. Varni, Ph.D. to assess health related quality of life in pediatric populations. The 23-item scales were developed to measure the core dimensions of health as defined by the World Health Organization (WHO), and the measurement model consists of developmentally appropriate forms for children by age. The measurement must include both child self-report and parent proxy-report and has been widely used in pediatric health outcomes evaluation. Reliability estimates for the core scales in both the self- and proxy-report are greater than the .70 standard, and validity has been demonstrated in known groups comparisons and correlations with other measures of disease burden. Parents will complete the parent proxy instrument for children aged 2-18. Children aged 5-7 will complete the PedsQL Young Child Report. Children ages 8-12 will complete the PedsQL Child Report, and children ages 13-18 will complete the PedsQL Teen Report. These instruments are designed to be self-administered.

- **Medical Home Family Index** – an instrument developed by the Center for Medical Home Improvement to measure patient/caregiver satisfaction with the care received from their primary care provider. This instrument is designed to be self-administered.

- **Utilization Questionnaire- an instrument developed by the Technical Working Group of the SCDTDP for the specific information needs of this study that could not be met with pre-existing instruments. This questionnaire collects information on the demographic characteristics of the patients enrolled in the SCDTDP, their health/disease status, and their use of health care services and SCD treatments. This instrument is designed to be administered by an interviewer who will collect**

the information either through an interview with the patient or caregiver (if patient is a minor), the medical record, or project database. Items that cannot be obtained through medical records or a project database will be obtained through an interview conducted during a regularly scheduled office visit.

Exhibit 2.1: Data Collection Instruments, Target Population, Sample and Mode of Administration

Data Collection Method	Target Population	Sample	Administration
SF-36	Adults (persons aged ≥18) with SCD	280	Self administered
PedsQL	Parents/c of minors (less than 18 years) with SCD	120	Self administered
PedsQL	Children and Adolescents	100	Interviewer (ages 5-7) Self administered (ages 8-18)
Medical Home Family Index	Parents of minors with SCD Adults with SCD	400	Self administered
Health Care Utilization Questionnaire	Parents of minors with SCD Adults with SCD	400	Interviewer administered

Quality Assurance

The NCC will provide training for the data collection staff approximately one to two months prior to the initiation of the data collection. The training topics will cover the procedures for explaining the study and the consent process, instructing participants on the completion of the forms and providing instruction on completing individual items on each of the forms. The NCC will train on procedures for verifying the correct record, assuring data security, and standardized conventions for completing the form.

In addition to training data collection staff, the NCC will develop a control system that will be the primary mechanism for tracking the flow of data and the progression of activities throughout the study. The control system will generate reports on the number of forms completed

by Networks and serve as the repository for the data once it has been uploaded to NCC servers. Other quality control procedures will also be implemented:

- internal data editing specifications to detect problems such as out of range responses;
- generation of frequencies on a periodic basis to detect any outliers or data inconsistencies that may require special attention; and
- monitoring reports to facilitate discussion with the Network sites during periodic conference calls regarding any issues of performance or compliance.

3. Methods to Maximize Response Rates

The focus of the NCC will be to provide the Networks with technical assistance in the recruitment, retention and monitoring of their patient sample. A response rate of 80% or better is feasible for this study for a number of reasons. First, the majority of the participants to be enrolled in this study have been under the care of the Network providers for a number of years. SCD is rare in the population at large and requires a unique and complex set of treatments and therapies; therefore, patients tend to remain in one geographic region for an extended period of time and establish relationships with a specific set of providers. Having a pre-existing relationship with the patients to be enrolled in the SCDTDP will facilitate recruitment and retention. There are subpopulations such as immigrants or adolescents transitioning into adult care who may be more transient and therefore difficult to locate for follow-up. These individuals will be identified early on and Networks will work closely with them to transition them to new providers if they should move away from the Network's area and maintain up-to-date contact information.

Second, the nature of SCD and the intensity of the SCDTDP interventions require the Networks to maintain frequent contact with their SCD patients (at least every other month). This frequency

of contact will also diminish the risk of loss to follow-up as contact information will be regularly updated during these contacts.

Third, a similar study of persons with SCD sponsored by HRSA, the Sickle Cell Disease and Newborn Screening Program, achieved its target sample of 300 participants and was able to retain 100% of these individuals through the completion of the study.

4. Tests of Procedures

As noted earlier, the SF-36 ®, the PedsQL®, and the Medical Home Family Index have been previously used in other research and their psychometric properties rigorously evaluated (Ware, Snow, Kosinski 1992, Varni, Limbers, Burwinkle, 2007, Cooley et. al, 2003)**.** Reference articles on these instruments are provided as separate documents.

The Utilization Questionnaire is a new form developed for this study. It was cognitively tested in April 2008, with 9 participants. The findings from that test indicated necessary revisions in a few of the elements – expanding the range of educational choices, adding other symptoms and complications, and the deletion of self-report as a source of immunization status.

5. Statistical Consultants

The data collection and analysis will be conducted by staff of the Research Triangle Institute. Names of key staff are listed below:

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