Supporting Statement
Sickle Cell Disease Treatment Demonstration Program

A. Justification

1. Circumstances of Information Collection

This statement is a request for Office of Management and Budget (OMB) approval for an evaluation of the Sickle Cell Disease Treatment Demonstration Program (SCDTDP). The purpose of the evaluation is to determine the effectiveness of the demonstration projects in achieving important health and quality of life outcomes for the sickle cell disease clients and their families served through the SCDTDP Networks. This is a new activity.

In 2004 Congress enacted and the President signed into law P.L. 108-357, the American Jobs Creation Act of 2004. Section 712 of P.L. 108-357 authorized a demonstration program for the prevention and treatment of Sickle Cell Disease. The legislation was enacted to (1) create an optional medical assistance program for individuals with Sickle Cell Diseases for treatment and education, genetic counseling and other services to prevent mortality and decrease morbidity from Sickle Cell Disease, and (2) to create a demonstration program, the SCDTDP, under the Health Resources and Services Administration (HRSA).

The SCDTDP provides grants to federally-qualified and nonprofit health care providers to establish geographically distributed regional networks that will work with comprehensive Sickle Cell Disease centers and community-based support organizations

to provide coordinated, comprehensive, culturally competent, and family-centered care to families with Sickle Cell

Disease. In FY06, HRSA's Maternal Child Health Bureau (MCHB) awarded four Network grants to the Illinois Sickle Cell Association Network; Alabama Network for Sickle Cell Care, Access, Prevention, and Education; Carolina Partnership for Sickle Cell Treatment Continuum of Care; and the Cincinnati Sickle Cell Network. For further details regarding this program, see attachment A.

The SCDTDP represents an innovative application of the medical home model by developing systematic and coordinated networks to meet the complex and multi-faceted needs of a patient population with chronic health needs. The term "medical home" refers to a partnership approach with families to provide access to quality health care in a cost effective manner in the primary care setting. The criteria for a medical home include the following: having (1) a usual place for sick/well care, (2) a personal health provider, (3) no difficulty in obtaining needed referrals, (4) needed care coordination, and (5) family/person centered care.7 (See attachment B for information on medical home model.)) The SCDTDP fits squarely with the broader goals of the MCHB. Specifically, it supports three of the Bureau's five goals:

- Goal 3 – Eliminate Health Barriers and Disparities;
- Goal 4 – Improve the Health Infrastructure and Systems of Care; and
- Goal 5 – Assure Quality of Care.

Under the authorizing legislation, a National Coordinating Center (NCC) was established for the demonstration program to: (1) collect, coordinate, monitor, and report on best practices and findings regarding the activities of the demonstration program; (2) identify

a model protocol for eligible entities with respect to the prevention and treatment of Sickle Cell Disease; (3) identify educational materials regarding the prevention and treatment of Sickle Cell Disease; and, (4) prepare a final report on the efficacy of the demonstration program based on evaluation findings. It is for this final report that this evaluation is being conducted.

2. Purpose and Use of Information

As part of the NCC mandate to collect, coordinate, monitor, and report on best practices and findings regarding the activities of the demonstration program, the SCDTDP Networks will collect health services utilization, health status, patient satisfaction and quality of life assessments from the demonstration clients during two phases of their participation – at baseline and 12 months post intervention. The purpose of this data collection will be to evaluate the effectiveness of the demonstration projects in achieving important treatment outcomes for the patients served. Specifically, the SCDTDP seeks to answer the following:

- *Do individuals with Sickle Cell Disease (SCD) enrolled in the SCDTDP receive appropriate treatment and genetic counseling; experience reduced morbidity; fewer hospitalizations and Emergency Room (ER) visits; and report improved capacity to manage the disease and satisfaction with care?.*

The data to be collected will provide HRSA with information it will use to assess the merit of the demonstration program and the benefit of replicating the model to other SCD provider sites. The collection of health and utilization data will greatly enhance the

ability of the SCDTDP to demonstrate whether or not the project achieved its legislative intent.

3. Use of Improved Information Technology

The data will be collected by the Networks through medical record abstractions and self-report using interviews and hard copy questionnaires. This information collection effort does not lend itself to the use of information technology for the purpose of reducing respondent burden. Overall, the grantees do not have the technology available for the purposes of electronic data collection; therefore, the grantees will be providing paper submission to the NCC.

4. Efforts to Identify Duplication

The SCDTDP is a unique demonstration program and there are no other available sources of data to address the requirements of the legislation. Where it is feasible and data sharing agreements and proper patient authorizations can be obtained, data will be abstracted from the medical record. These data, however, represent only a subset of the health status and healthcare utilization information needed for the evaluation.

5. Involvement of Small Entities

This activity does not have a significant impact on small businesses or small entities.

6. Consequences if Information Collected Less Frequently.

Data are to be collected at baseline during the patient's enrollment period into the project and again 12 months after the baseline assessment. This is the minimum number of data collection points needed to assess change in health status or utilization of health services.

7. Consistency with the Guidelines in 5 CFR 1320.5(d) (2)

The proposed data collection complies fully with 5 CFR 1320.5(d)(2)

8. Consultation Outside the Agency

The notice required in 5 CFR 1320.8(d) was published in the *Federal Register* on January 4, 2008, on pages 870-871. No comments were received.

The NCC convened a Technical Working Group comprised of representatives of the five Networks as well as expert SCD scientists and researchers for the purpose of ensuring that the data collection would be 1) relevant and appropriate and 2) of minimum burden to the patients and the organizations administering the data collection forms. A roster of the Technical Working Group members is presented below in Exhibit 8.1. Over the course of seven months (from July 2007 to January 2008), the Technical Working Group met monthly to develop a uniform data collection instrument (Individual Utilization Questionnaire) and to identify other instruments currently available and in use in SCD research that would meet the evaluation needs of the project.

Exhibit 8.1. SCDTDP Technical Working Group Members

Organization	Contact Person	Role	Phone Number Fax Number	E-mail
University of Alabama-Birmingham	Roy McDonald, MPH	Data Manager	205-939-5849 Fax: 205-939-9571	rmcdonald@peds.uab.edu
University of Alabama-	Thomas Howard, M.D.	Hematologist, Education	205-939-9285 205-939-1941	THoward@peds.uab.edu

Birmingham		Director		
University of Cincinnati	Thomas Webb, MD, MSc	PI	513.556.2870 513.401.8890 (pager)	thomas.webb@uc.edu webbta@ucmail.uc.edu
University of Cincinnati	Annette Lavender, RN	Nurse Practitioner	513-584-0373 513-584-0369	lavendar@UCMAIL.UC.EDU
Stedman-Wade	Dr. Kweku Laast, MD, MPH	PI	910.483.6694	klaastmd@aol.com
Fayetteville State University	Akbar Aghajanian Ph.D.	Director, Research Center for Health Disparities	910-672-2927 Fax: 910-672-1068	aaghajanian@uncfsu.edu
Christian Community Health Center (CCHC)	Linda D. Drawhorn, MS, RN	Project Coordinator	773-298-2051 773-233-8542	Linda.drawhorn@cchc-rchm.org
SCDAI	Valerie Beckley, MSW	Executive Director SCDAI	312-345-1100	valerie.beckley@mgci.com valeriebeckley@comcast.net
Univ of NC at Greensboro	Joseph Telfair, DrPH, MSW, MPH	Project Director	336-334-4777p 336-334-3238f 336-404-5377(mobile)	j_telfai@uncg.edu
Meharry Medical College (MMC)	Maria del Pilar Aguinaga, PhD, CLDir(NCA)	Clinical Advisor	615-327-6591 615-327-6593	maguinaga@mmc.edu
HRSA-MCHB	Judith Hagopian MPH/MSW	Project Officer	301-443-5698p 301-443-8604f	jhagopian@hrsa.gov
HRSA-MCHB	R Lorraine Brown RN, BS	Project Officer	301-443-9775p 301-443- 8604f	lbrown@hrsa.gov
National Heart, Lung and Blood Institue/Univ of Colorado Health Sciences Cntr	Kathryn Hassell, MD	Technical Working Group Chairperson	303-372-9071	hassellk@nhlbi.nih.gov Kathryn.Hassell@UCHSC.edu
Research Triangle Institute (RTI)	Marian Sullivan, MS, MPH	Project Manager	301-230-4677p 301-230-4646f	msullivan@rti.org
RTI	Lisa Newman, MSPH	Assistant Manager	301-230-4652p 301-230-4646f	lnewman@rti.org
RTI	Lucia Rojas-Smith, DrPH	Implementation & Evaluation Task Leader	202-728-2053 202-728-2095f	lucia@rti.org
RTI	Jutta Thornberry, BA	Data Collection Manager	202-230-4645 301-230-4646f	jps@rti.org
RTI	Brenda Stone-Wiggins, MPH	Technical Assistance Coordinator	919-316-3328	bwiggins@rti.org

9. Remuneration of Respondents

Respondents will not be remunerated.

10. Assurance of Confidentiality

Personally identifiable information of patients will not be collected in this evaluation. Responses reported in final reports will be reported in aggregate only.

11. Questions of a Sensitive Nature

There are no questions of a sensitive nature.

12. Estimates of the Annualized Hour Burden

Data will be collected using four different instruments: the Utilization Questionnaire, the SF-36 for adults aged 18 and over; the PedsQL for adolescents and children and their parents and; the Medical Home Family Index. The total burden estimate per participant is shown below: **Exhibit 12.1**:

Exhibit 12.1 Estimated Burden Hours

Type of Respondent	Form Name	Number of Respondents	Responses per Respondent	Total Responses	Hours per Response	Total Burden Hours
Sickle Cell Disease clients or parents	Utilization Questionnaire (pre-demonstration)	400	1	400	.75	300
Sickle Cell Disease clients or parents	Utilization Questionnaire (post demonstration)	400	1	400	.50	200
Sickle Cell Disease clients	SF-36 Health Survey for adults over 18 years of age	280	2	560	.25	140
Parents of Sickle Cell Disease Clients (age	PedsQL for parents of children & adolescents 18 years or younger*	120	2	240	.25	60

0-18 years)						
Sickle Cell Disease Child and Adolescent Clients (aged 5 to 18 years)	PedsQL for children & adolescents 18 years or younger*	100	2	200	.25	50
Sickle Cell Disease clients or caregivers	The Medical Home Family Index (Health Care Satisfaction)	400	2	800	.25	200
Total		500		2,600		950

* Only one form is completed by respondent based on age and responder.

The total burden is 950. This would be the maximum estimated level of burden since some of the demonstration networks will be able to abstract medical records for some of the data collected on the Utilization Questionnaire.

The estimates of burden for the SF-36, the PedsQL, and the Medical Home Family Index were derived from published estimates of administering these instruments. [1] (References are provided separately.) The Utilization Form was pre-tested in April 2007 with 9 respondents and the estimates of burden are based on the results of those assessments.

The costs to the patients and caretakers completing the data collection are presented in **Exhibit 12.2** and are based on the average hourly earnings of production and non-supervisory workers on private, non-farm payrolls published by the Department of Labor in March 2008 (http://www.bls.gov/news.release/pdf/realer.pdf).

Exhibit 12.2. Annualized Cost to Respondents

[1] Sf-36 : http://www.rand.org/health/surveys_tools/mos/mos_core_36item.html; PedsQL: http://www.pedsql.org/about_pedsql.html, Medical Home Family Index: http://www.medicalhomeimprovement.org/outcomes.htm

Type of Respondent	Number of Respondents	Frequency of Response	Average U.S Hourly Wage Rate	Respondent Cost
Person with SCD or Caretaker	400	6	17.80	$ 42,720

13. Estimates of Annualized Cost Burden to Respondents

There are no capital or start-up costs for this evaluation. There are no operation or maintenance costs for respondents.

14. Annualized Cost to the Federal Government

HRSA selected a contractor to conduct the evaluation at a cost of approximately $600,000 annually as well as the cost for one FTE GS-13 at 20% time at $20,000 to monitor the project. The total annual costs for this collection of information are approximately $620,000.

15. Changes in Burden

This is new data activity.

16. Time Schedule, Publication and Analysis Plans

Project Time Table

Data collection will be conducted over an 18 month period beginning on approximately October 1, 2008, pending OMB approval. A final report presenting the findings of the evaluation of the demonstration program will be presented at the conclusion of the study.

Additional manuscripts for publication in peer reviewed articles may be prepared in accordance with the decisions of the Technical Workgroup. Exhibit 16.1 below presents the proposed timeline for the data collection.

Exhibit 16.1 Project Timeline

Activity	**Time Schedule**
Patient Enrollment	October 2008
Field Questionnaires	October 2008-December 2009
Complete Field Work	December 2009
Validation	January-February 2010
Analyses	March 2010-July 2010
Final Report/Manuscript	September 2010

Planned Data Analyses

The purpose of the planned analysis is to describe the characteristics of the patients served by the SCDTDP and to assess whether the program has had any demonstrable effect on access to treatment (e.g. hydroxyurea therapy) and genetic counseling, health care utilization (outpatient visits, hospitalization and emergency room visits), morbidity (pain and physical functioning) and patient satisfaction with care. To achieve these analytic goals the planned analysis will involve a mix of descriptive, bivariate, and regression analyses.

Patients enrolled in the SCDTDP will be characterized by their demographics (e.g. age, gender, and race), economic status, and type of Sickle Cell disease. All the patients will have baseline and a follow-up data. Categorical or ordinal variables such as gender and race will be summarized by frequency distribution. Continuous variables such as age and number of nights stay in the hospital during the past 12 months will be summarized by mean, median, standard deviation, minimum and maximum value. Outliers and possible data errors will be detected for further formal statistical analysis.

We recognize that there are important concerns regarding the issue of missing data and the potential that missing values can lead to misleading results. We will review non-response data as well as item-non-response to minimize the possibility of reaching invalid and insignificant results. A concern is that the assumptions behind many statistical procedures are based on complete cases, and missing values can complicate the theory required. We will review mean, standard deviation, frequencies, number of missing and non-missing values, number of extreme values for all variables, and we will examine data from several different angles using different diagnostic reports to understand the missing data. We will conduct missing value analysis to find if the cases with missing values are systematically different from cases without missing values.

Based on our initial missing value analysis, we may impute some missing values. The Lead Statistician and the Technical Working Group will develop a list of *Key Data Elements* that will be eligible for imputation if the missing values exceed 5%. Multiple imputation (MI) techniques will be employed. MI techniques are commonly accepted methods for replacing missing values with imputed values based on the underlying model. We will use MI techniques to replace missing values with imputed values based on the underlying model and assuming the missing values are 'missing at random' (MAR) defined by Little and Rubin (1987). We will choose the imputation model approximately compatible with the analyses to be performed on the imputed datasets to eliminate bias (Meng (1995) and Rubin (1996). We will impute the missing values m times ($m \geq 5$) and create m complete data sets. We will analyze each of the m completed data sets separately and integrate the m analysis results into a final result.

To explore the crude association of an outcome variable with a single factor, we will use bivariate analysis. Selection of statistical methods for bivariate analysis will depend on the measurements of two variables. Cross-tabulation will be used when both variables are categorical. Chi-square or if the frequency of the data is too small - Fisher's exact test in the case of categorical data; t-test and analysis of variance (ANOVA) will be used to compare the continuous outcomes. We may use nonparametric methods like Wilcoxon rank-sum test for ordinal data, as this test is appropriate for correlated samples as we have

here. Simple correlation coefficients will be used to describe the association between two continuous or interval variables such as education and household income level.

Dependent Variables: Specifically, the evaluation of the SCDTDP will examine the following health outcomes as presented in **Exhibit 16.2** as well as others not listed here.

Exhibit 16.2 Illustrative Dependent Variables	
Variable	**Type**
Health care Utilization	
Physician visits	Continuous
ER visits	Continuous
Hospital stays	Continuous
Length of hospital stays	Continuous
Hydroxyurea therapy	Categorical
Genetic counseling	Categorical
Health Outcomes	
Pain	Categorical
Pneumonia or Acute Chest Syndrome	Categorical
Fever	Categorical
Severe Infection	Categorical
Stroke	Categorical
Kidney Damage	Categorical
Gall Bladder Attack	Categorical
Spleen Problems	Categorical

Eye Damage	Categorical
Leg Ulcers	Categorical
Priapism	Categorical
Hand Foot Syndrome	Categorical
Physical and Socioemotional Function	
Summary scores for physical functioning	Continuous
Summary scores for emotional functioning	Continuous
Summary scores for social functioning	Continuous
Summary scores for school functioning (child and adolescents only)	Continuous
Patient satisfaction with care	
scored mean of questions related to communication, office responsiveness, care plan continuity and family involvement	Continuous

A set of illustrative tables of the study outcomes (health care utilization health outcomes, patient quality of life, and patient satisfaction with health care) are presented in **Exhibits 16.3 to 16.6** below.

Exhibit 16.3 Health Care Utilization Encounters at Baseline and 12 Months Post Intervention

Type of Health Care Encounter	**Number of Encounters Baseline**	**Number of Encounters Post Intervention at 12 months**
Primary Care Office Visit		
Specialist Office Visit		
Eye Care Visit		
Emergency Room Visit		
Overnight Hospital Stay		
Genetic Testing and/or Counselling Visit		

Exhibit 16.4 Complications of SCD at Baseline and 12 Months Post Intervention

Type of SCD Complication	**Number and % Reporting Baseline**	**Number and % Reporting Post Intervention at 12 months**
Pain		

Pneumonia or Acute Chest Syndrome		
Fever		
Severe Infection		
Stroke		
Kidney Damage		
Gall Bladder Attack		
Spleen Problems		
Eye Damage		
Leg Ulcers		
Priapism		
Hand Foot Syndrome		

Exhibit 16.5 Quality of Life Measures at Baseline and 12 Months Post Intervention

Quality of Life Domain	Baseline Score	Post Intervention at 12 months
Physical Summary Score		
Psychosocial/Mental Summary Score		

Exhibit 16.6 Health Care Satisfaction Outcomes at Baseline and 12 Months Post Intervention		
Health Care Satisfaction Variable	**Number and % Often or Always** **Baseline Score**	**Number and % Often or Always** **Post Intervention at 12 months**
Care available when needed including holidays, weekends, evenings		
Primary care physician uses helpful ways to communicate		
Primary care physician has staff who help with referrals, payment issues or follow-up activities		
Office staff available to review medical record when asked		
Office providers know about condition, history or other concerns and priorities		
Primary care office sponsors activities to support family		
Primary care providers asks patient/caregiver to share knowledge and expertise		

We will also perform statistical modeling on the patient data to examine the relationship between specified variables addressed in the authorizing legislation and participation in the SCDTDP. According to the nature or measurement of outcome variables, different

regression methods will be used to examine the relationship between specific factors and an outcome of interest while controlling for potential confounding factors.

The seven main hypotheses that will be used to specify the statistical modeling are linked to the research question specified in Section 2: *Do individuals with Sickle Cell Disease (SCD) enrolled in SCDTDP receive appropriate treatment and genetic counseling; experience reduced morbidity; fewer hospitalizations and Emergency Room (ER) visits; and improved capacity to manage the disease and satisfaction with care?*

- ***Hypothesis 1***. *The patients enrolled in the SCDTDP are more likely to receive Hydroxyurea Therapy in the 12 months following enrolment compared to the 12 month period preceding enrolment after controlling for possible confounding demographic and health factors.*

- ***Hypothesis 2***. *The patients, caregivers, and family members enrolled in the SCDTDP likely to receive genetic counseling in the 12 months following enrolment compared to the 12 month period preceding enrolment after controlling for possible confounding demographic and health factors.*

- ***Hypothesis 3***. *The patients enrolled in the SCDTDP experience fewer emergency room visits in the 12 months following enrolment compared to the 12 month period preceding enrolment after controlling for possible confounding demographic and health factors.*

- ***Hypothesis 4***. *The patients enrolled in the SCDTDP experience fewer emergency room visits in the 12 months following enrolment compared to the 12 month period preceding enrolment after controlling for possible confounding demographic and health factors.*

- ***Hypothesis 5***. *The patients enrolled in the SCDTDP experience fewer inpatient hospital stays in the 12 months following enrolment compared to the 12 month*

period preceding enrolment after controlling for possible confounding demographic and health status factors.

- ***Hypothesis 6****. The patients enrolled in the SCDTDP experience less pain in the 12 months following enrolment compared to the 12 month period preceding enrolment after controlling for possible confounding demographic and health status factors.*

- ***Hypothesis 7****. The patients enrolled in the SCDTDP report improved patient satisfaction with health care services following enrolment compared to the 12 month period preceding enrolment after controlling for possible confounding demographic and health status factors.*

A considerable limitation of the planned analysis is the lack of a control group – although a baseline and follow-up measures will be collected for each patient. This limits our ability to robustly evaluate whether the SCDTDP activities are definitively responsible for improvements in outcomes. Ideally a control group would be selected from the patient base served by the SCDTDP, however, as this is a service grant, the services are offered to all patients with SCD. Those who decline or refuse services could potentially serve as a control group but they may be different in known and unknown ways that would make comparisons to the intervention group problematic. Moreover, the number of SCD patients who would refuse the services would likely be too small to yield a sufficiently large sample size and collecting a comparable control group from clinical sites outside of the demonstration region is beyond the resources of this project. With these limitations in mind, whatever results are demonstrated by this project will not be generalizeable to the larger population of SCD patients but pertain only to those directly served by the project. Nonetheless, we believe the findings, although suggestive, will help inform the manner in which services for persons with SCD are coordinated and organized within a complex health care environment.

17. Exemption for Display of Expiration Date

The expiration date will be displayed.

18. Certifications

This information collection fully complies with the guidelines in 5 CFR 1320.9. The necessary certifications are included in the package.