

Supporting Statement

Sickle Cell Disease Treatment Demonstration Program - Quality Improvement Data Collection

OMB Control No. 0906-XXXX

Terms of Clearance: None

A. Justification

1. Circumstances Making the Collection of Information Necessary

This statement is a request for Office of Management and Budget (OMB) approval for the quality improvement data collection strategy of the Sickle Cell Disease Treatment and Demonstration Program (SCDTDP). The purpose of the quality improvement data collection strategy is to meet the goals of the SCDTDP and to implement a system to monitor the progress of Maternal and Child Health Bureau funded activities in improving care and health outcomes for individuals living with sickle cell disease who utilize care delivered by the SCDTDP network sites. 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.¹ The legislation was enacted to (1) create an optional medical assistance program for individuals with sickle cell disease 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 under the direction of the Health Resources and Services Administration (HRSA). Please refer to Appendix A for a copy of the America Jobs Creation Act of 2004. Section 712 of P.L. 108-357.

The SCDTDP is funded and administered by the Genetic Services Branch of the Division of Services for Children with Special Health Needs in the Maternal and Child Health Bureau (MCHB), Health Resources and Services Administration (HRSA). MCHB provides grants to federally-qualified and nonprofit health care centers to establish geographically distributed regional networks that are working 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 and sickle cell trait.

Under the authorizing legislation, a National Coordinating Center (NCC) was also established via a HRSA contract 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.

¹ 108th Congress of the United States of America. American Jobs Creation Act of 2004. (Bill no H.R. 4520) Washington, DC: 108th Congress of the United States of America, 2004. Available at: <http://thomas.loc.gov/cgi-bin/bdquery/z?d108:H.R.4520>:

The importance of this program is evident in the fact that individuals with SCD suffer significant morbidities such as pain episodes, acute chest syndrome, and stroke.² In addition, adults experience additional complications secondary to SCD including renal disease, cognitive impairment due to strokes, and unexplained sudden death.

Access to high quality care profoundly affects outcomes for people with SCD. Specific technical advances such as penicillin prophylaxis, vaccines, broad spectrum antibiotics,^{3,4} blood transfusion protocols and Transcranial Doppler screening^{5,6} as well as improved supportive care have contributed to dramatic improvements in life expectancy from 14 years in the mid-1970s to over 40 years in the mid-1990s.^{7,8} More recently, hydroxyurea (HU), the only FDA-approved therapy for SCD,^{9,10} has been shown to lower sickle cell related complications such as pain crises and acute chest syndrome, along with associated ED visits and hospitalizations.¹¹ HU both improves quality of life for patients and lowers overall costs of care.¹² Although outcomes have improved, much remains to be done. More than one in ten (14%) children with homozygous SCD (HbSS) die before the age of 18,^{13,14} with substantial geographic variation in childhood outcomes.^{15,16} Adults with SCD have less access to and overall lower quality of care than do children.^{17,18} Many adults with SCD don't have access to hematologists specializing in SCD nor to primary care clinicians with appropriate

² Davis H, Gergen PJ, Moore RM 1997.

³ Brousseau D, Owens P, Mosso A, Panepinto J, Steiner C. Acute care utilization and rehospitalizations for sickle cell disease. *JAMA* 2010; 303:1288.

⁴ National Institutes of Health. National Heart, Lung and Blood Disease Institute and Division of Blood Diseases and Resources. The management of sickle cell disease. NIH Publication (No. 02-2117), 4th edition. Bethesda, MD; 2002.

⁵ American Academy of Pediatrics Section on Hematology/Oncology Committee on Genetics. Health supervision for children with sickle cell disease. *Pediatrics* 2002;109:526-35.

⁶ Shekelle P, Chassin M, Park R. Assessing the predictive validity of the RAND/UCLA appropriateness method criteria for performing carotid endarterectomy. *Int J Technol Assess Health Care* 1998;14:707-27.

⁷ Shekelle P, Kahan J, Bernstein S, Leape L, Kamberg C, Park R. The reproducibility of a method to identify the overuse and underuse of medical procedures. *N Engl J Med* 1998;338:1888.

⁸ West S, King V, Carey T, et al. Systems to rate the strength of scientific evidence. Rockville, MD: Agency for Healthcare Research and Quality; 2002.

⁹ National Institutes of Health 2002.

¹⁰ Charache S, Terrin ML, Moore RD, et al. Effect of hydroxyurea on the frequency of painful crises in sickle cell anemia. *N Engl J Med* 1995;332:1317-1322.

¹¹ Wang WC, Oyeku SO, Luo Z, et al. Hydroxyurea is associated with lower costs of care of young children with sickle cell anemia. *Pediatrics*. 2013; 132(4): 677-83.

¹² *Ibid.*

¹³ West S, King V, Carey T, et al 2002.

¹⁴ Davis H, Schoendorf KC, Gergen PJ, Moore RM Jr. National trends in the mortality of children with sickle cell disease, 1968 through 1992. *Am J Public Health*. 1997 Aug;87(8):1317-22.

¹⁵ Davis H, Schoendorf KC, Gergen PJ, Moore RM Jr. Geographic differences in mortality of young children with sickle cell disease in the United States. *Public Health Rep*. 1997 Jan-Feb;112(1):52-8

¹⁶ McGlynn E, Damberg C, Kerr E, Schuster M. *Quality of Care for Children and Adolescents*. Santa Monica, CA: RAND; 2000.

¹⁷ Haywood C Jr, Beach MC, Lanzkron S, et al. A systematic review of barriers and interventions to improve appropriate use of therapies for sickle cell disease. *J Natl Med Assoc* 2009; 101:1022-1033.

¹⁸ Maxwell K, Streeby A, Bevan D. Experiences of hospital care and treatment seeking for pain from sickle cell disease: Qualitative study. *BMJ* 1999; 318: 1585-1590.

knowledge and expertise. Adult use of HU varies greatly from region to region and provider to provider and is generally highly underutilized.^{19,20,21,22}

The SCDTDP was created to address these issues and provides a unique opportunity to measure quality of care in these settings in order to develop strategies to improve care and outcomes for patients with SCD. There are three specific aims for the program: (1) increase the number of providers treating persons with sickle cell disease, (2) increase the number of providers prescribing hydroxyurea, and (3) increase the number of providers knowledgeable about treating sickle cell disease as well as increase the number of sickle cell patients that are seen by providers knowledgeable about sickle cell disease.

2. Purpose and Use of Information Collection

Measurement overview:

The SCDTDP will use the collective impact framework and quality improvement (QI) approaches. These approaches rely on the use shared measurement and reporting on the impact of changes on key process and outcome measures at the regional, state and local levels. These measures will consist of outcome measures such health care utilization. An example of an outcome measure is the number of providers in a state who had seen a SCD patient in the last 12 months.

Collection of these QI measures is integral to the improvement processes and the data will be used to:

1. Monitor and drive improvement. The data will allow teams to determine if the changes they are testing are leading to improvement and enable the NCC to provide the regional coordinating center with feedback on their performance and improvement.
2. Identify changes that have proven most effective at improving care for individuals with SCD. The most effective changes can then be spread throughout the network of partners and stakeholders.
3. Refine a common model protocol with respect to the prevention and treatment of sickle cell disease.
4. Provide HRSA/Congress information on overall progress of the program.

The SCDTDP data collection strategy incorporates measures developed through a rigorous evidence-based process. It will be implemented through a customized data entry and reporting system, called the Collaboratory, built expressly to support this work. Measures were developed based on the aims of the project, a literature review, the

¹⁹ Lanzkron S, Haywood C Jr, Fagan PJ, Rand CS. Examining the effectiveness of hydroxyurea in people with sickle cell disease. *J Health Care Poor Underserved*. 2010 Feb;21(1):277-86. doi: 10.1353/hpu.0.0272.

²⁰ Brawley OW, Cornelius LJ, Edwards LR, Northington Gamble V, Green BL, Inturrisi C, James AH, Laraque D, Mendez M, Montoya CJ, Pollock BH, Robinson L, Scholnik AP, Schori M. National Institutes of Health Consensus Development Conference Statement: hydroxyurea treatment for sickle cell disease. *Ann Intern Med*. 2008; 148: 932-8.

²¹ Wang WC, Oyeku SO, Luo Z, et al. 2013.

²² Ritho J, Liu H, Hartzema AG, Lottenberg R. Hydroxyurea use in patients with sickle cell disease in a Medicaid population. *Am J Hematol*. 2011 Oct;86(10): 888-90. doi: 10.1002/ajh.22134. Epub 2011 Aug 22.

measurement strategies proposed by the regional coordinating centers, and expert opinion. All measures were evaluated by content experts and a group of grantee representatives (See Appendices B and C). Once finalized, measures were translated to a QI data collection and entry form that regional coordinating centers will use to submit data. A data request template was developed for the regional coordinating centers to submit to the State Medicaid and Medicaid Managed Care Organization (MCO) offices to ensure uniform data collection. A database was developed to calculate the data entered and create reports all within in the Collaboratory system.

Currently, QI data are collected from State Medicaid and Medicaid MCOs on a quarterly basis by four SCDTDP grantees to monitor and drive improvements in care intended to affect outcomes and to inform collaborative activities. The data being pulled is generated through claims submitted for treatment of patients with sickle cell disease. This data is held by state Medicaid offices that are mandated to collect information on procedural and diagnostic claims data to submit to the Centers for Medicare and Medicaid Services (CMS). Once the SCDTDP grantees request aggregate data on provider encounters and prescriptions of hydroxyurea for patients with sickle cell disease, this information is stored in local databases within each RCC. The request being made for the purposes of this OMB application, are for the four SCDTDP grantees to complete one QI data collection and entry form for each State Medicaid and/or Medicaid MCOs in their region quarterly to submit for the NCC's final report. This aggregate data, subset by state, will be entered into an online data collection portal, the NICHQ Collaboratory. From this platform, the NCC will be able to generate run charts for comparisons of the quality indicators across states. The number of data pulls being requested will be limited as the end of the grant period is approaching. The number of quarterly requests will be no more than three quarters and any baseline data the grantees are able to submit with their initial data draw. A general overview of the QI form grantees are asked to submit is described below. More detailed information on the data collection items and instrument are provided in Appendices D through F.

We anticipate submitting a revision to this request at a future time to add a small subset of additional measures in order accommodate and reflect the changing needs and lessons learned through these efforts (See Appendices G and H).

Data collection procedures and instruments:

Each of the regional coordinating centers will complete the QI data collection and entry form on a quarterly basis throughout the duration of the program. Data elements on the form will be collected via data request to State Medicaid and Medicaid MCO offices in their region and directly entered into a secure, web-based data collection tool, the Collaboratory, by a data lead from each of the regional coordinating centers.

- The QI data collection and entry form will be used to collect aggregated percentage data on the number of providers treating SCD patients and providers prescribing hydroxyurea within the plan (i.e., State Medicaid or Medicaid MCO). Over time, this information will provide an overall view of providers in the regional networks.

By reporting on a quarterly basis, the SCDTDP grantees (respondents) are able to track and reassess their performance on quality measures. In addition, they are able to receive timely feedback on their performance across the regional network. This is done with the goal of monitoring and driving improvements in outcomes for individuals with sickle cell disease. Specific details about calculation of burden hours, for the QI instrument used, are outlined in Section 12.

Participant size (the 4 grantees):

The respondents for the QI data collection effort will be the data lead from each of the four regional coordinating centers. These individuals will submit data received from State Medicaid and Medicaid MCO offices based on the data request form that specifies the numerators, denominators, exclusions, and other details relevant for the claims data. Respondents are being asked to submit data quarterly from all State Medicaid and Medicaid MCOs within their region. The SCDTDP grantees are continually building relationships and submitting requests to State Medicaid and Medicaid MCOs, so the number of data submissions they receive each quarter will vary and possibly expand. The burden hours have therefor been calculated, below, as an average to express the lowest and highest possible number of data sources that each region may receive. Specific details about calculation of burden hours and the ranges for the QI instrument used are outlined in Section 12.

Analysis:

Aggregated data (numerators/denominators) obtained from insurance claims submitted to State Medicaid and Medicaid MCO offices will be entered directly into the Collaboratory, which will be a secure web-based data collection tool. The data entered into the Collaboratory will be analyzed via the internal database that will export the QI outcome measures to be viewed in time series chart format by regional teams and the NCC to track performance.

The NCC will use these measures to assess the regional coordinating centers' performance and improvement on quality measures. In the context of the SCDTDP, regional coordinating centers are conducting regional QI collaboratives with state partners to improve local systems and processes to reliably deliver evidence based care to patients with SCD. The regional coordinating centers, as well as the project faculty and leadership, will rely on these quality measures to assess the reliability of care, and the degree of improvement achieved by the regional coordinating centers. In addition, the data collected will be necessary for the preparation of the annual report to HRSA, as well as the final Congressional report detailing the results of this program as required by federal legislation.

3. Use of Improved Technology and Burden Reduction

The goal of the SCDTDP is to help regional coordinating centers improve care and outcomes for individuals living with SCD. In an effort to attain these goals and reduce respondent burden, an electronic data and reporting system will be utilized.

Using a web based QI data collection and entry form, the SCDTDP regional coordinating centers will submit aggregated claims data gathered via a data request from State Medicaid and Medicaid MCOs. The data will be used to inform the regional coordinating centers' work across their regional collaboratives and with their state level partners. The web based data collection tool will utilize secure interfaces.

The online data and reporting system, the Collaboratory, is Federal Information Security Management Act (FISMA) compliant and meets all of the HRSA IT Security Protocol requirements.

4. Efforts to Identify Duplication and Use of Similar Information

The SCDTDP is a unique demonstration program and there are no other available sources of data that provide the opportunity to improve care and outcomes in a manner timely enough to inform the QI initiatives of the regional coordinating centers. While State Medicaid and Medicaid MCOs submit these data to CMS, the delay can be more than 18 months, which makes the data not timely enough for a three-year project. Hence we are asking for these data to be submitted from the State Medicaid offices and Medicaid Managed Care Organizations to the regional coordinating centers directly so that they can use the quarterly reports to inform their improvement activities. We have inquired of national CMS directly about the availability of these data from them, and their files are only up to date for the states included in the regions for this project as of 2011.

To ensure that the work is not duplicated, the Medical Director of the National Coordinating Center attends many relevant national meetings, including National Heart Lung and Blood Institute National Blood Disorder Coordinating Committee meeting, Sickle Cell Disease Association of America (SCDAA) annual convention, American Society of Hematology meeting, and other ad hoc meetings with other federal and non-federal entities focused on SCD. Moreover, the Medical Director and HRSA Program Officer consult with other federal agencies, providing a bi-directional interface between the SCDTDP and relevant national efforts to both accelerate improvements and avoid duplication.

An environmental scan of existing literature yields few quality indicators for individuals with sickle cell disease. The quality indicators chosen were synthesized from current evidence and vetted by content experts. We attempted to align these quality measures with the proposed quality measures from each regional coordinating center to meet the overall aims of the project.

5. Impact on Small Businesses or Other Small Entities

No small businesses will be involved in this study.

6. Consequences of Collecting the Information Less Frequently

Quality Improvement data will be collected on a quarterly basis throughout the duration of the program.²³ Less frequent assessments will not provide sufficient timely feedback to inform program design, activities and improvements nor the required data specified by the legislation noted above. Also, there are no legal obstacles to reduce the burden.

7. Special Circumstances Relating to the Guidelines of 5 CFR 1320.5

The request fully complies with the regulation.

8. Comments in Response to the Federal Register Notice/Outside Consultation

8A. The 60-day Federal Register Notice required by 5 CFR 1320.8(d) was published in the Federal Register on December 28, 2015, vol. 80, No. 248; pp. 80777-80778 (see Appendix I). No comments were received.

8B. The SCDTDP data collection strategy incorporates measures developed through a rigorous evidence-based process and implemented through a common, standardized data entry and reporting system built expressly to support this work. The measures were established using a process that included a comprehensive literature review followed by discussion and rating at a Data Summit and further review by project faculty and Oversight Steering Committee members. The names, titles and contact information for both the participants in the Data Summit and the project Oversight Steering Committee are listed in Appendices B and C.

9. Explanation of any Payment/Gift to Respondents

Respondents will not be remunerated or compensated.

10. Assurance of Confidentiality Provided to Respondents

No data collected through this project will contain personally identifiable data as only aggregated claims data will be collected. In addition, data will be kept private to the extent allowed by law.

11. Justification for Sensitive Questions

There are no questions of a sensitive nature being asked of respondents.

12. Estimates of Annualized Hour and Cost Burden

Exhibit 12.A summarizes the estimated hour burden of data collection. Data will be collected using one electronic QI data collection and entry form: the SCDTDP Data Form. The total burden estimate range per participant is shown below. (see Appendix J for further details and calculation rationale).

²³ Institute for Healthcare Improvement. The Breakthrough Series: IHI's Collaborative Model for Achieving Breakthrough Improvement. *Diabetes Spectrum*. April 2004; 17(2):97-101.

Form Name	Number of Respondents	Total Number of Responses per Respondent	Average Number of Responses per Respondent	Total Burden Hours	Average Burden per Response (in hours)
SCDTDP Data Form	4	3	17.5	105	26.25
Total	4	3	17.5	105	26.25

The cost to the regional coordinating centers' data leads gathering the data from State Medicaid and Medicaid MCOs and submitting into the online data collection platform is presented in Exhibit 12.B. Each regional grantee will receive data from every state in their region as a response, for an average of 17 responses per respondent. Since we are towards the end of this grant, we anticipate, possibly, 2-3 quarters of data submission from each SCDTDP grantee. The burden cost is based on the average hourly wage rates from the 2011 National Occupational Employment and Wage Estimates from the Bureau of Labor Statistics for all occupations available at:

http://www.bls.gov/oes/current/oes_nat.htm

Exhibit 12.B Estimated Annualized Burden Costs			
13. Type of Respondent	Average Burden Hours	Hourly Wage Rate	Total Respondent Costs
Regional Coordinating Center Data Lead	105	\$40.60	\$4263.00
Total	105	\$40.60	\$4263.00

Estimates of other Total Annual Cost Burden to Respondents or Record keepers/Capital Costs

There are no capital or startup costs associated with data collection.

14. Annualized Cost to Federal Government

The cost of the contract for the Sickle Cell Disease Treatment Program is approximately \$871,738 over three years, for an annualized cost of \$290,579.33. This includes both staff time as well as associated technology costs for this project.

15. Explanation for Program Changes or Adjustments

This is a new data collection.

16. Plans for Tabulation, Publication, and Project Time Schedule

Project Time Table

Data collection will be conducted over a 12 month period beginning approximately in August 2016, pending OMB approval. A final report presenting the findings of the

evaluation of the demonstration program will be presented at the conclusion of the project. Manuscripts for publication in peer reviewed journals may be prepared depending on the decisions of the Data Team. Exhibit 16.A below demonstrates the proposed timeline for the QI data collection.

Exhibit 16.A Project Timeline	
Activity	Time Schedule
Receipt of OMB approval	Estimated August 2016
SCDTDP Data Collection and Entry Forms Utilized	August 2016
Implementation of NCC data collection protocols	August 2016
Data submission	Quarterly (November 2016) through the funded period 2014-2017
Data Analysis and Technical Assistance	Quarterly (November 2016) through the funded period 2014-2017
Annual Report to HRSA/MCHB	Annual (October) through the funded period 2014-2017
Final Report to HRSA/MCHB	September 2017
Congressional report	September 2017

Planned Data Analyses

Planned data analyses will be primarily descriptive in nature. We will assess the frequency of outcome measures such as health care utilization (e.g., hydroxyurea prescriptions). We will use quarterly percentages to display the frequency distribution of the measures of interest. Those quarterly percentages, displayed in a time series graph (run chart), reflect the work of the team to improve care processes and outcomes.

For improvement purposes, additional data analyses based on principles of statistical process control will also be performed.²⁴ The aim of the analysis is to assess the stability and predictability of the process of care that generates the measure. For example, the measure “Percent of providers who have seen a patient with SCD twice within the last 12 months” responds to the efforts of local improvement teams within regional networks to design processes for ensuring that the numbers of providers treating individuals with SCD is increasing. Run charts and control charts will be used to assess whether the process is working consistently, and thus producing predictable results. In addition, these analytic methods will be used to assess the degree of improvement against the null hypothesis of no improvement.

17. Reason(s) Display of OMB Expiration Date is Inappropriate

The expiration date will be displayed.

18. Exceptions to Certification for Paperwork Reduction Act Submissions

There are no exceptions to the certification.

²⁴ Benneyan JC, Lloyd RC, Plsek PE. Statistical Process Control as a tool for research and healthcare improvement. *Qual Saf Health Care*. 2003;12:458-464

List of Appendices

Appendix A: America Jobs Creation Act of 2004. Section 712 of P.L. 108-357.

Appendix B: SCDTDP Data Summit Participants

Appendix C: SDCTDP Oversight Steering Committee Members

Appendix D: SCDTDP Data Collection Form

Appendix E: SCDTDP Measure Specifications

Appendix F: SCDTDP Data Request Form

Appendix G: Tracking Project Performance Measures Template

Appendix H: Project Performance/Outcome Measure Detail Sheet Template

Appendix I: SCDTDP OMB 60-day FR Notice

Appendix J: Justification for Annual Estimate of Burden