Supporting Statement

Sickle Cell Disease Treatment Demonstration Regional Collaborative Program:

Quality Improvement and Performance Measure Data Collection

OMB Control No. 0906-XXXX

**Terms of Clearance:** None

## A. Justification

### 1. Circumstances Making the Collection of Information Necessary

The Health Resources and Services Administration (HRSA) is requesting that the Office of Management and Budget (OMB) review and approve the Quality Improvement (QI) and Performance Measures (PM) data collection forms for the Sickle Cell Disease Treatment Demonstration Regional Collaborative Program (SCDTDRCP). The purpose of the data collection strategy is to evaluate the effectiveness of the SCDTDRCP and how the program can improve the coordination of service delivery for individuals with sickle cell disease (SCD), train health professionals to increase access to quality care, and collaborate with various stakeholders to optimize health outcomes for individuals with SCD. Data collected by the SCDTDRCP grantees will be summarized in a Report to Congress. This is a new activity.

Individuals with SCD suffer significant morbidities such as pain episodes, acute chest syndrome, and stroke.[1] In addition, adults experience complications secondary to SCD including renal disease, cognitive impairment due to strokes, and unexplained sudden death.[2] Access to high quality care profoundly affects outcomes for people with SCD. Specific technical advances such as penicillin prophylaxis, vaccines, broad spectrum antibiotics,[3,4,5] blood transfusion protocols and Transcranial Doppler (TCD) screening [6,7] as well as improved supportive care have contributed to dramatic improvements in life expectancy. Despite these treatment advances, life expectancy for individuals with SCD is still 20-30 years lower than the general US population.[8,9]

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[1] Davis H, Gergen PJ, Moore RM 1997.

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Piel FB, Steinberg MH, Rees DC. Sickle Cell Disease. N Engl J Med. 2017 Apr 20;376(16):1561-1573.

[3] Ibid

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

[5] 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.

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

[7] 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.

[8] Paulukonis ST, Eckman JR, Snyder AB, et al. Defining Sickle Cell Disease Mortality Using a Population-Based Surveillance System, 2004-2008. Public Health Rep. 2016;131(2):367-375.

[9] Lanzkron S, Carroll CP, Haywood Jr. C. Mortality rates and age at death from sickle cell disease: U.S., 1979-2005. Public Health Rep. 2013;128(2):110-116.

Hydroxyurea (HU), an FDA-approved therapy for SCD,[10,11] has been shown to lower sickle cell related complications such as pain crises and acute chest syndrome, along with associated ED visits and hospitalizations.[12] HU both improves quality of life for patients and lowers overall costs of care.[13] Newer disease modifying therapies, including FDA approved L-glutamine[14] and recently approved voxelotor[15,16] and crizanlizumab[17], also have been shown to decrease sickle cell related complications. Although outcomes have improved, gaps in quality and access to care still exist. [18,19,20,21,22]

The Sickle Cell Disease Treatment Demonstration Regional Collaborative Program (SCDTDRCP) was first authorized in 2004 and reauthorized under the Sickle Cell Disease and Other Heritable Blood Disorders Research, Surveillance, Prevention and Treatment Act of 2018, 42 USC § 300b-5. Please see Appendix A. The statute requires that HRSA continues efforts, including by awarding grants, to develop or establish mechanisms to improve the treatment of SCD, and to improve the prevention and treatment of complications of SCD in populations with a high proportion of individuals with SCD.

The goals of the SCDTDRCP are to:

- Improve health outcomes in individuals with SCD;
- Reduce morbidity and mortality caused by SCD;
- Reduce the number of individuals with SCD receiving care only in emergency departments; and
- Improve the quality of coordinated and comprehensive services to individuals with SCD and their families.

The program currently funds five grantees to establish a regional network of state and local partners and provide leadership and support for regional and statewide activities. The grantees

[10] National Institutes of Health 2002.

[11] 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.

[12] 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.

[13] Ibid.

[14] Niihara Y, Miller ST, Kanter J, Lanzkron S et.al on behalf of Investigators of the Phase 3 Trial of l-Glutamine in Sickle Cell Disease. A Phase 3 Trial of l-Glutamine in Sickle Cell Disease. N Engl J Med. 2018 Jul 19;379(3):226-235.

[15] Blair HA Voxelotor: First Approval. Drugs. 2020 Feb;80(2):209-215.

[16] Vichinsky E, Hoppe CC, Ataga KI et.al. on behalf of HOPE Trial Investigators. A Phase 3 Randomized Trial of Voxelotor in Sickle Cell Disease. N Engl J Med. 2019 Aug 8;381(6):509-519.

[17] Blair HA. Crizanlizumab: First Approval. Drugs. 2020 Jan;80(1):79-84.

[18] 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.

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

[20] Oyeku SO, Faro EZ. Rigorous and Practical Quality Indicators in Sickle Cell Disease Care. *Hematology Am Soc Hematol Educ Program*. 2017 Dec 8;2017(1):418-422.

[21] Kanter J, Kruse-Jarres R. Management of sickle cell disease from childhood through adulthood. Blood Rev. 2013;27(6): 279-287. doi:10.1016/j.bire.2013.09.001.

[22] Lee L, Smith-Whitley K, Banks S, Puckrein G. Reducing Health Care Disparities in Sickle Cell Disease: A Review. Public Health Rep. 2019 Nov/Dec;134(6):599-607.

develop and create systemic mechanisms to improve the prevention of complications and treatment of SCD, by: 1) increasing the number of providers treating individuals with SCD using the National Heart, Lung and Blood Institute (NHLBI) Evidence-Based Management of Sickle Cell Disease Expert Panel Report; 2) using tele-mentoring, telemedicine and other provider support strategies to increase the number of providers administering evidence-based sickle cell care; and 3) developing and implementing strategies to improve access to quality care with emphasis on individual and family engagement/partnership, adolescent transitions to adult health care, and care in a medical home.

The authorizing statute also requires HRSA to administer a contract for a National Coordinating Center (NCC) for the demonstration program that conducts the following activities: (1) collect, coordinate, monitor and distribute data as well as report on best practices and findings regarding the activities of the demonstration program; (2) identify a model protocol (standardized set of procedures, materials and tools to improve sickle cell care) for eligible entities with respect to the prevention and treatment of complications of SCD; (3) identify educational materials regarding the prevention and treatment of complications of SCD; and, (4) prepare a final report summarizing the demonstration program based on evaluation findings.

## 2. Purpose and Use of Information Collection

The purpose of the QI and PM data collection is to evaluate the effectiveness of the SCDTDRCP and how the program can improve the coordination of service delivery for individuals with sickle cell disease, train health professionals to increase access to quality care and collaborate with various stakeholders to optimize health outcomes for individuals with sickle cell disease. Collection of the QI and PM measures will be used to:

1. Inform the progress of regional QI initiatives. The data will allow grantees to monitor whether the changes they made are associated with improvements in SCD care.
2. Show areas of clinical care that are robust or in need of improvement with regard to: HU prescription rates; prophylactic vaccination rates; TCD rates, transition planning and ECHO attendance.
3. Refine a common model protocol with respect to the prevention and treatment of SCD.
4. Provide information on the overall progress of the program to HRSA and for the statutorily-required report to Congress.

The QI and PM data measures for the SCDTDRCP were identified based on a review of published literature, NHLBI's guidelines, and national priorities. The measure specifications were created and refined by a group of grantee representatives and experts (Appendix B).

### Quality Improvement (QI)

All five SCDTDRCP grantees implement QI initiatives to improve the quality of SCD treatment and access to care. Each grantee also works with and supports local sites (i.e., university, medical center, etc.) that provide SCD care within their region to implement QI initiatives.

All the grantees and local sites are required to implement initiatives to increase the HU use and conduct one or more additional QI initiatives on the following topics: pneumococcal vaccinations, TCD screening, and transition planning.

The QI data collection tool includes the following measures:

- HU Use[23]
    - Percentage of patients prescribed HU 9 months to less than 18 years old
    - Percentage of patients prescribed HU 18 years old and over
- TCD rates
    - Percentage of eligible patients (ages 2-16) who had a TCD screening within the last 15 months
- Pneumococcal vaccination status
    - Percentage of SCD patients who are up to date with individual and/or all recommended pneumococcal vaccinations
- Transitions to Adult Care
    - Percentage of patients between 14 and 17 years of age with documented education on transition to adult health care.

Using the data collection tool, the grantees and local sites will collect data on a quarterly basis on applicable measures depending on which QI initiatives they are undertaking. The data will be extracted from patients' charts either via chart reviews or electronic health records. The local sites will send their data to the grantees using an excel spreadsheet or by entering data into a database form (e.g. REDCap) of their choice developed by the grantee. The grantees will aggregate their own data and the data received from the local sites and submit the aggregate data to the NCC via the data entry portal of CoLab. Please see Appendix C for a sample QI data collection and entry form that a local site may use and a copy of the QI data collection and entry form for the grantees to submit to the NCC.

Performance Measures (PM)

In order to understand SCD care provided and the reach of the SCDTDRCP activities across regions, the following PM have been established:

- Number of providers receiving the survey in the grantee's region.
- Number of SCD patients seen by a provider in the past year
- Number of providers participating in telementoring activities in the past year
    - Percentage of above providers comfortable treating patients with SCD
- Number of providers that saw at least one SCD patient in the past year
    - Percentage of above providers that prescribed HU
- Number of SCD patients Prescribed HU in the past year

The five SCDTDRCP grantees will send a survey once a year to providers they work with within their region who provide care to SCD patients to collect PM data. These providers may or may not practice at the local sites where QI data is collected from. Once the providers complete the survey, the grantees will aggregate the individual responses and submit the PM data to the NCC via the data entry portal of CoLab. Please see appendix D for a copy of the provider survey and the PM data entry form for the grantees.

[23] In light of the recently approved therapies for SCD, the QI measure includes questions related to use of disease modifying therapies to account for these secular trends.

By collecting QI data on a quarterly basis and PM data on an annual basis, small to large changes can be seen. Because there are no existing data sets that show SCD care on a national level, having a solid initial data set and regular follow-ups is important to drive change for improved outcomes for individuals with SCD.

Quarterly QI data analyses will include descriptive statistics, both regional and in aggregate form. The NCC will assess the frequency of outcome measures such as HU prescriptions by quarter. The NCC will use quarterly percentages to display the frequency distribution of the measures of interest. Analyses will be aggregated both across quarters and by region to descriptively explore regional and temporal variation within the data set. Analysis of annual PM survey data will include frequencies. Tables and charts will be utilized as needed to assess provider data across multiple years and among regions.

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**

In an effort to attain these goals and reduce respondent burden, all data will be collected electronically. Local sites will determine the least burdensome methodology to collect and submit QI data to the grantees, whether it is via excel spreadsheet or an electronic database. The provider survey for PM data will be administered using an online platform and the responses will be collected electronically, unless a provider requests a paper copy. The grantees will also aggregate and submit QI and PM data electronically to NCC. The web-based data collection tools will utilize secure interfaces and the online data and reporting system the grantees will utilize, CoLab, is Federal Information Security Management Act compliant and meets all of the HRSA IT Security Protocol requirements.

**4. Efforts to Identify Duplication and Use of Similar Information**

The SCDTDRCP is a unique demonstration program, and the data collected are unique to the program. There are no other available sources of data that provide this comprehensive of a data set that reflects the work being done in the care of patients living with SCD at a regional and national level. The quarterly collection of QI data will create a robust baseline of information to be able to see changes over time.

Efforts are made by both the NCC and the HRSA Project Officer to ensure that information collection is not duplicated. These efforts are also intended to accelerate improvements in SCD outcomes and provide a bi-directional interface between the SCDTDRCP and relevant national activities. These efforts include:

- The NCC and the HRSA Project Officer attend national conventions (e.g., Sickle Cell Disease Association of America), SCDTDRCP Oversight Steering Committee (OSC) meetings, American Society of Hematology meetings;
- The NCC and the HRSA Project Officer consult with other federal agencies, specifically, the Office of Minority Health who is heavily engaged in pursuing high-quality, comprehensive care for SCD patients;

- The NCC and the HRSA Project Officer attend additional ad hoc meetings with other federal and non-federal entities focused on SCD.

An environmental scan of existing literature yields few quality indicators for individuals with SCD. The QI and PM data measures were chosen by synthesizing information from current evidence and vetted by the grantees and content experts.[24] All measures are aligned with the overall aims of the project.

**5. Impact on Small Businesses or Other Small Entities**

Physicians are asked to complete the provider survey for PM data. In order to minimize burden, survey completion is only requested annually and only asks questions absolutely required to establish PM data.

**6. Consequences of Collecting the Information Less Frequently**

QI data will be collected on a quarterly basis throughout the duration of the program and PM data will be collected annually. 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.

**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**

**Section 8A:**

The 60-day Federal Register Notice was published in the Federal Register on January 23, 2020, Vol. 85, No. 15; pp. 3935-37 (Appendix E). There were no public comments. In an effort to derive a realistic burden estimate for the reporting requirements, the SCDTDRCP grantees were contacted. Their names and contact information are provided in the table below.

**Section 8B:**

The SCDTDRCP QI and PM data measures for the project were identified based on review of published literature, NHLBI's guidelines and national priorities. The measure specifications were created and refined by a group of grantee representatives, the NCC, and content experts. These groups convened to listen to presentations of possible measures, engage in discussion, and then participate in final selection. Meetings and calls were held in 2017 and 2018. The names, titles, and contact information for these groups are listed in Appendix B.

In addition, as stated above, the SCDTDRCP grantees were contacted in May 2018 to gather input from the grantees and the local sites they work with on potential burden as well as QI data collection methodology, number of respondents, frequency of data collection, and expected data collection capacity. Feedback from the grantees and the NCC were reviewed and considered in finalization of data collection tools, strategy, and burden estimate calculations. The grantees were also recently consulted to integrate any updates in burden estimates in the final burden

[24] Oyeku SO, Faro EZ. Rigorous and Practical Quality Indicators in Sickle Cell Disease Care. *Hematology Am Soc Hematol Educ Program*. 2017 Dec 8;2017(1):418-422.

calculation. The names and contact information of individuals that participated in the consultation is listed in the chart below.

| Name | Contact Information |
|---|---|
| Rosalyn Steward, Co-Director of Northeast SCDTDRCP at Johns Hopkins University | rstewart@jhmi.edu |
| Sophie Lanzkron, Co-Director of Northeast SCDTDRCP at Johns Hopkins University | slanzkron@jhmi.edu |
| Bailey House, Data lead of Northeast SCDTDRCP at Johns Hopkins University | bailey.house@jhmi.edu |
| John Strouse, Co-Director of Southeast SCDTDRCP at Duke University | john.strouse@duke.edu |
| Ify Osunkwo, Co-Director of Southeast SCDTDRCP at Atrium Health | Ify.Osunkwo@atriumhealth.org |
| Julie Kanter, Co-Director of Southeast SCDTDRCP at University of Alabama | jkanter@uabmc.edu |
| Allison King, Co-Director of Heartland SCDTDRCP at Washington University | King_A@kids.wustl.edu |
| Taniya Varughese, Data lead of Heartland SCDTDRCP at Washington University | eostow@wustl.org |
| Lisa Shook, Co-Director of Midwest SCDTDRCP at Children's Hospital Medical Center | lisa.shook@cchmc.org |
| Bennett Farrell, Data lead of Midwest SCDTDRCP at Children's Hospital Medical Center | Christina.Bennett@cchmc.org |
| Marsha Treadwell, Co-Director of Pacific SCDTDRCP at University of California San Francisco | marsha.treadwell@ucsf.edu |
| Ashley Fraser, Data lead of Pacific SCDTDRCP at University of California San Francisco | ashley.fraser@ucsf.edu |
| Kim Sprunck, Project Director of the NCC at National Institute for Children's Health Quality | ksprunck@nichq.org |
| Barbara Lambiaso, Project Manager of the NCC at National Institute for Children's Health Quality | blambiaso@nichq.org |
| Bill Adams, Informatics Expert for the NCC | bill.adams@bmc.org |
| Suzette Oyeku, Medical Expert for the NCC | Soyeku@montefiore.org |

**9. Explanation of any Payment/Gift to Respondents**

Respondents will not receive any payments or gifts.

**10. Assurance of Confidentiality Provided to Respondents**

All data collected for this project is done so in accordance to protocols of the grantees and local sites and all data sent to the NCC is aggregate, deidentified data. No data that HRSA receives contains personally identifiable information and the 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**

**12A. Estimated Annualized Burden Hours**

| Form Name | Number of Respondents | Number of Responses per Respondent per Year | Total Responses per Year | Average Burden per Response | Total Burden Hours per Year |
|---|---|---|---|---|---|
| **SCDTDRCP Quality Improvement Measures*** | 55 | 4 | 220 | 13 | 2,860 |
| **SCDTDRCP Performance Measures** | 305 | 1 | 305 | 1 | 305 |

| | | | | | |
|---|---|---|---|---|---|
| **Total** | 360 | | 525 | | 3,165 |

*Note: Total burden hours per year shown represents the maximum number of estimated hours. Actual hours may be lower since many of the respondents may not be collecting data all QI initiatives.

Quality Improvement (QI)

The respondents for the QI measures include the five SCDTDRCP grantees and approximately fifty local sites they work with. All the respondents will collect QI data quarterly. Because the QI data collection requires initial pre-work in order to set up database or process, this pre-work burden estimate was distributed to each burden per response equally over 3 years of data collection (12 responses). The burden estimates per response, based on consultations described in Section 8, vary because some local sites (approximately 15) collect data via chart reviews and while others (approximately 35) pull data from electronic health records. In addition to data collection, the five grantees have additional burden of aggregating the collected data and submitting to the NCC. A weighted average of these burden estimates was calculated for the average burden per response. As noted above, these calculations were done on the basis that all 55 respondents will collect data on all four QI initiatives so the actual burden hours may be lower as many of the respondents currently are implementing two or three QI initiatives.

Performance Measures (PM)

The respondents for the PM data collection include the five SCDTDRCP grantees and approximately 300 providers they work with. The 300 providers will respond to the provider survey administered annually and the five grantees will complete the survey as well as aggregate and report the PM data to the NCC. Similarly to QI data, the burden per response represents a weighted average of the burden estimates for the individual providers and the five grantees.

**12B. Estimated Annualized Burden Costs**

The salary of physicians varies significantly across states and by specialty. While the majority of the respondents for both QI and PM data will be physicians, some may be other types of care providers, such as nurse practitioners or physician assistants. Some of the QI data collection also may be done by additional support staff, such as nurses or informaticists. The estimated cost was calculated based on the mean hourly wage for physicians from the National Occupational Employment and Wage Estimates from the Bureau of Labor Statistics for all occupations available at: https://www.bls.gov/bls/blswage.htm.

| Type of Respondent | Total Burden Hours per Year | Hourly Mean Wage Rate | Total Respondent Costs |
|---|---|---|---|
| Physicians | 3,165 | $97.81 | $309,569 |
| **Total** | 3,165 | | $309,569 |

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

Other than their time, there is no cost to the respondents.

**14. Annualized Cost to Federal Government**

Approximately 50% of the NCC contract for the SCDTDRCP accounts for activities related to this data collection, which is approximately $250,000 per year. This includes both staff time as well as associated technology costs for this project. In order to oversee the contractor and provide technical assistance to the SCDTDRCP grantees, the federal staff, who is both the Contracting Officer's Representative and Project Officer, spends 15% time, at a cost of $21,837 (GS 14 Step 7 on OPM's Salary Table 2020: https://www.opm.gov/policy-data-oversight/pay-leave/salaries-wages/salary-tables/pdf/2020/DCB.pdf). The total annual cost to the Federal Government for the QI and PM data collection is $271,837.

**15. Explanation for Program Changes or Adjustments**

This is a new information collection.

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

The QI and PM data will be collected over three years, beginning in Summer/Fall 2020, pending OMB approval. The QI data will be collected quarterly. The local sites and the grantees will have about six weeks to aggregate and submit data that cover the previous quarter. For example, the data submission period of QI data for Q3 2020 (July 1 through September 30, 2020) will be October 1 through November 15, 2020. The provider survey will be sent out on May 1st of each year and will remain open for six weeks. Once the survey closes, the grantees will have four weeks to aggregate survey results and submit PM data to the NCC.

Data analyses will include quarterly QI data and annual PM survey metrics. QI data analyses will include descriptive statistics, both regional and in aggregate form. The NCC will assess the frequency of outcome measures such as HU prescriptions by quarter. The quarterly percentages will be used to display the frequency distribution of the measures of interest. Analyses will be aggregated both across quarters and by region to descriptively explore regional and temporal variation within the data set. Analysis of annual provider survey data will include frequencies. Tables and charts will be utilized as needed to assess provider data across multiple years and among regions.

The NCC will prepare a Report to Congress presenting the findings of the evaluation of the demonstration program at the conclusion of the current project cycle of SCDTDRCP, which ends on August 31, 2021. Manuscripts for publication in peer reviewed journals may be prepared depending on the decisions of the NCC.

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

The OMB number and Expiration date will be displayed on every page of every form/instrument.

**18. Exceptions to Certification for Paperwork Reduction Act Submissions**

There are no exceptions to the certification.

**List of Appendices**

Appendix A: Sickle Cell Disease and Other Heritable Blood Disorders Research, Surveillance, Prevention and Treatment Act of 2018, 42 USC § 300b-5

Appendix B: SCDTDRCP Quality Improvement Measures Data Collection Documents

Appendix C: SCDTDRCP Performance Measures Data Collection Documents
Appendix D: SCDTDRCP OMB 60-Day FRN Published.1.23.20