Executive Summary

Overview of Sickle Cell Disease

Approximately 100,000 Americans live with sickle cell disease (SCD). One out of every 365 African American children are born with SCD each year, making it one of the most common serious genetic disorders in the United States[1]. For much of the 20th century, SCD was considered a pediatric condition, as individuals with SCD did not survive into adulthood. However, with medical advancements and improvements in care, most people with SCD are now living into adulthood. Despite these gains, people living with this illness face a lifelong battle with pain, infection and other serious health problems that can affect every organ in the body. Those with the most severe form of the disease can expect to live 20-30 fewer years than the average American.[2]

Access to high quality healthcare is critical to prevent complications and early mortality related to this disease, yet many people with SCD are unable to get the care they need. One example is access to hydroxyurea (HU), the only FDA-approved drug to treat SCD until the recent approval of L-glutamine in July 2017. Unfortunately, HU is often under-prescribed to those who could benefit from it most. This is due to concerns among both providers and patients about potential toxicity of this treatment, making some providers uncomfortable with prescribing this medication.[3] Exacerbating potential issues with access to proven treatments are the limited number of medical centers across the country that specialize in treating SCD and the reality that most people with SCD do not live near these centers. Patients with SCD also often seek care in emergency departments (EDs), which is costly, or from primary care providers (PCPs) who do not have experience treating the disease. Expanding access to care and increasing the pool of knowledgeable providers, including PCPs, for people with SCD will improve and lengthen their lives.

PURPOSE & SCOPE

Given the severity of SCD and recognizing the need for adequate resources to address the structural and societal barriers to improving the care of those living with the disease, Congress enacted legislation authorizing the Health Resources and Services Administration (HRSA) to carry out the Sickle Cell Disease Treatment Demonstration Program (SCDTDP). The SCDTDP works to improve the care of patients with SCD with a goal of improving long-term outcomes (e.g., decrease early mortality) and short-term outcomes (e.g., increased usage of HU). Over the past 13 years, the SCDTDP's activities have included training medical providers, building models of care to meet the evolving needs of people living with SCD, and supporting the transition from pediatric care to adult care.

During the 2014-2017 funding cycle of the SCDTDP, the National Institute for Children's Health Quality (NICHQ) was selected to serve as the National Coordinating Center (NCC) for four SCDTDP-funded Regional Coordinating Centers (RCCs): the Heartland, the Midwest, the Northeast, and the Pacific. Each of the RCC grantees were tasked with working with states to develop innovative regional networks of care and to increase provider knowledge around SCD to support the appropriate care and treatment of SCD. In its role as NCC, NICHQ led several network-wide measurement activities and assisted in the coordination and sharing of best practices for improvement work done in each of the RCCs.

Table below shows the SCDTDP geographical areas along with the estimated SCD populations to be reached in each region.

TABLE 1:
Regional Structure of SCDTDP

RCC	LEAD ORGANIZATION	STATES/ TERRITORIES	APPROXIMATE # OF INDIVIDUALS LIVING WITH SCD
Heartland	Washington University, St. Louis	MO, IA, KS, NE	2,620
Midwest	Children's Hospital Medical Center	IL, IN, MI, MN, OH, WI	15,000
Northeast	Johns Hopkins University	MD, VA, DE, District of Columbia, NJ, NY, PA, PR, USVI, WV	32,000
Pacific	Center for Comprehensive Care and Diagnosis of Inherited Blood Disorders	CA, ID, OR, WA, NV, AK, HI, AZ, Pacific Basin	7,100
Estimated total number of patients with SCD included in project regions			56,720

The three primary aims of the 2014-2017 SCDTDP were to:

- Improve access to care by increasing the number of providers treating patients with SCD;
- Increase the number of providers prescribing HU; and
- Increase the number of patients with SCD that are receiving care from providers knowledgeable about treating SCD.

The 2014-2017 Sickle Cell Disease Treatment Demonstration Program represented the first time in U.S. history that improvements in sickle cell disease care were tackled on a large, regional and national scale.

MEASUREMENT STRATEGY

The measurement strategy for the project included both a national and regional focus. NICHQ assisted regional teams throughout the project in their efforts to collect, analyze, and share data to identify progress towards achieving the program's aims. The measurement efforts for this SCDTDP grant cycle (2014-2017) began with a data summit in November 2014. During this summit, HRSA, the NCC, key SCD experts, and the RCCs identified shared objectives and a common measurement strategy. This work was guided by key lessons learned from the previous SCDTDP (2010-2013), also lead by NICHQ, and focused on creating a larger measurement strategy that allowed for broader state and regional level assessment of patient care and provider practice within key populations.

This summit brought together key representatives from the four RCCs with the goal of getting input from experts in the field to identify potential barriers to accessing and aligning data across the regions.

While various data sources were considered, including local registries, electronic health data, and administrative data, the following data streams were prioritized for the SCDTDP:

- Administrative Data: Aggregate data from Medicaid-managed care organizations (MCOs) and Medicaid claims data from state-level Medicaid departments
- Minimum Data Sets (MDS): Individual patient-level data from surveys and electronic health records (EHRs)

To support this shared measurement strategy, NICHQ, serving as the NCC, first developed a detailed set of measure specifications that included computable measure definitions and a data dictionary to ensure each region was collecting data the same way across regions. The data dictionary was used to inform the development of a web-based collaboration tool ("The Collaboratory" or "CoLab") to support rapid entry of aggregate data across the regions and the sharing of best practices and communication across regions. For confidentiality and privacy reasons, the NCC was only allowed to collect and share aggregate data (numerators and denominators).

The Administrative Data was identified as the source most likely to be able to capture data across the very large population among the four RCCs. Specific measures were developed related to assessing the number of providers providing, and patients with SCD receiving, consistent care (Aim 1) as well as provider HU-prescribing behaviors and patient behavior around filling HU prescriptions (Aim 2).

In parallel with the national administrative data work, the NCC and the RCCs also worked to develop local "registries" designed to capture a minimum set of data to allow the measurement of quality at the local level. This activity was called the Minimum Data Set (MDS) and allowed the RCCs to collect patient-level data from electronic health records (EHRs) and patient surveys to assess a larger number of quality measures. These measures mirrored the administrative measures, and focused on measures related to HU use (Aim 2) and patient access to providers and use of urgent care facilities, including in-patient and ER usage (Aim 1). The MDS enabled a more tailored and flexible system of local data collection that allowed patient care sites to track progress towards the three project aims as well as other improvements in quality of care. The MDS activity was a voluntary effort used locally and reported back to the NCC at the aggregate level.

The third aim of the project focused on improving provider knowledge of SCD. Because this information was a challenge to collect from the administrative or MDS data, the NCC developed program evaluation activities to better understand the provider education efforts across the regions. RCCs provided descriptions of state and regional provider education efforts as part of regular communication with the NCC. Additional information about the SCDTDP Measurement Strategy can be found in Section 2 of the report.

PROGRAM IMPACT AND ACCOMPLISHMENTS

Improving access: Increasing the number of clinicians seeing patients with SCD

Within each region, teams worked though the project period to improve access to care for both children and adults via a range of activities, such as opening comprehensive care centers in high-need locations as well as implementing patient outreach and follow-up with community health workers (CHWs) in collaboration with local SCD community-based organizations (CBOs). Section 3 of the report highlights this work. All four regions have made access to care a priority, and reported increasing the sites available to provide quality care for patients living with SCD. Nearly 11,000 patients with SCD received care from SCDTDP regional networks, reflecting an increase of more than 3,000 from baseline.

TABLE 2:

Growing Networks of Care

REGION	PACIFIC	HEARTLAND	MIDWEST	NORTHEAST
Baseline of total number of patients receiving care	1020	1723	1674	3168
% Increase in patients receiving care from baseline	24% (N= 1285)	3% (N= 1778)	20% (N= 1982)	85% (N= 5879)

Here are some examples of the strategies regions used to increase access to care for patients with SCD.

Heartland RCC - The Heartland Sickle Cell Disease Treatment Network established telementoring programs for healthcare providers to address geographic disparities in care access. For example:

- Nebraska implemented a telementoring program between the SCD Center for Excellence located at the Children's Hospital and Medical Center in Omaha and geographically distant providers so that patients and their local providers could have continued access to knowledgeable care between long distance visits with specialty providers.
- The Heartland regional lead set up a telementoring consultation practice with SCD care management teams at Centene, a large multi-state insurer running many Medicaid MCO plans across the region and other rural states.

Midwest RCC - STORM (Sickle Treatment and Outcomes Research in the Midwest) efforts have focused on helping systems address gaps that affect care. For example, when Illinois stopped accepting Medicaid patients from Indiana, there was a severe gap in access for patients in the city of Gary, IN, for which the closest geographic access to care had been Chicago, IL. Providers in Indiana filled this gap by setting up satellite clinics in Gary that connected patients and local providers to staff and resources at larger medical systems with SCD expertise across the state.

Northeast RCC - SiNERGe (Sickle Cell Improvement Across the NorthEast ReGion through Education) developed strong relationships with CBOs both in individual states and at the regional level to increase patient access to SCD care.

- CHW programs were able to identify adult SCD patients and connect them with a medical home for coordinated preventive care and specialty care to manage their disease progression and symptoms.
- The Northeast also established the first telementoring SCD ECHO® program,[4] a hub and spoke model of telementoring, which fosters education between medical providers to build skills through a structured curriculum and review of case reports. This program has created a community of practice to share and learn best practices, including providers across the region and also from the Midwest.

Telementoring and telehealth initiatives increased provider knowledge across the country. Nearly 100 Project ECHO®[5] clinics were held across regions, expanding opportunities for provider education for more than 200 providers.

Pacific RCC - PSCRC (Pacific Sickle Cell Regional Collaborative) saw increased patient access to care with the opening of two new comprehensive centers for SCD care in key urban areas targeting larger concentrations of patients with SCD:

- The MLK, Jr. Outpatient Center for Adults, with a catchment area of more than 1,000 potential patients with SCD, in Los Angeles, CA; and
- The Children's Specialty Center of Nevada in Las Vegas, which opened its doors to provide larger patient populations with access to knowledgeable physicians and systems of quality care for patients with SCD and their families.

Improving the Treatment of SCD: Increasing HU Use

The National, Heart, Lung and Blood Institute (NHLBI) recommends that all eligible patients with SCD should be offered or treated with HU.[5] Over the course of the three-year grant, all four SCDTDP regions reported increases in the number of patients with SCD being treated with HU within their partner sites. Section 4 of the report highlights these efforts. Increases in HU use have been attributed to improvements in patient access to care and improvements in care systems to better facilitate the integration of HU use in provider training and decision-making tools. Efforts to support both clinical decision-making as well as shared decision-making tools used with patients — such as patient brochures — are innovations that are beginning to show some impact. For example, the Pacific region saw an overall 26 percent increase of patients on HU after the release of the patient brochures.

All SCDTDP Regions Report Increases in HU Use

HEARTLAND:

- Among children, increased from 12% to 20%
- Among adults, increased from 14% to 17%

MIDWEST:

- Among all patients (pediatric & adult), increased from 48% to 69%
- Nearly 100% of all eligible patients now offered HU

NORTHEAST:

- Among children, increased from 23% to 34%
- Among adults, increased from 16% to 18%

PACIFIC:

- Among all patients, increased from 29% to 42%

Enhancing Care: Increasing the Number of Providers Knowledgeable about SCD

A key strategy to increase the number of providers available to care for patients with SCD is to increase the knowledge and clinical capacity of primary care clinicians. The four SCDTDP regional coordinating centers made great strides in increasing and improving provider education and knowledge about SCD treatment and care which are highlighted in Section 5 of the report. While there were many in-person events across the regions and states supported by local community efforts, teams also employed models of remote communication. Teams found online webinars and CME credit modules useful in reaching and educating providers.

The RCCs in the Northeast, Midwest and Pacific employed the innovative Project ECHO® (Extension for Community Healthcare Outcomes) Model[4] of telementoring and training. This collaborative approach to medical education and care management based at the University of New Mexico is designed to empower primary care clinicians to provide better care to more people with complex conditions where they live. This is one of the first models of an ECHO® program that looks at supporting providers to improve quality of care for a rare disease. This innovative model has provided a platform to provide best practice information to providers to help compensate for the gap in the quantity of knowledge-able providers. This has been especially critical for providers supporting care for adults with SCD, who not only require care for the disease, but whole patient care and care related to aging. Informed whole patient care will help improve patient health outcomes, enhance healthcare delivery and reduce costs overall.

LESSONS LEARNED

Key lessons learned for each of the three project aims are detailed below:

Aim 1: Increase number of providers treating patients with SCD

A challenging element for many teams was identifying providers willing to treat patients with SCD. To address this issue, teams had to be creative in their recruitment efforts. Experience from recruitment efforts suggests that:

- Often the providers most amenable to recruitment efforts are trainees.
- Partnerships of co-managing patient care between specialists in urban settings and primary care doctors in rural settings can create opportunities to improve care for patients living in geographically isolated areas.
- Many centers are most likely to add nurse practitioners as care providers for this population, particularly for adult patients.
- There is potential to increase access and linkages with providers who are knowledgeable about providing care for patients with SCD through the use of electronic media and social media (e.g., listings on the Heartland webpage).
- Redefining providers to be explicitly more inclusive (e.g., including nurses, nurse practitioners, CHWs) increases the scope and reach of recruitment efforts and reflects the reality that for many SCD centers of care, providers are often nurse practitioners, particularly for adult patients.

There was additional knowledge obtained on improving and innovating at the sites of clinical care, including:

- Inclusion of the voice of the community is critical to establishing early discussions with policymakers and decision-makers.
- Aligned electronic health record systems greatly facilitate communication and measure standardization, but this is rare and difficult to build into existing systems. For example, pediatric systems and adult systems vary greatly.

Consensus among the RCCs is that the opportunities and incentives to engage providers are very challenging. This requires programs to be flexible, adaptive and creative in thinking about their recruitment of providers who are willing to engage in SCD care and assist in increasing access to care.

Aim 2: Increase use of HU in treating SCD

For many of the regions, one of the key project impacts was strides made in increasing access to HU and increasing the number of providers willing to either prescribe or co-manage patients on HU. There were several lessons learned from programmatic efforts to increase HU use. Some of these include:

- Most providers lack the knowledge, confidence and decision-making tools to prescribe HU.
- Medical education webinars, publications and creation of provider education tools and materials to support HU prescription and management were able to increase provider comfort with prescribing HU.
- Specific tools and materials (e.g., brochures and roadmaps) directly related to HU's risks and benefits significantly helped increase the comfort level of providers around HU and their ability to explain these risks and benefits to their patients.
- Often the prescribers of HU are hematologists/oncologists rather than PCPs. Program efforts should focus on successful co-management between specialists and PCPs while also increasing knowledge of hematologists/oncologists in the overall care of people with SCD.
- For project results to be sustainable and best practices spread, providers need to be adequately reimbursed for caring for complex patients with SCD, many of whom have substantial social needs and declines in health due to complications of SCD co-occurring with the usual health problems of aging.
- Additional tools and interventions are needed to increase the number of providers prescribing HU.

With the right model of support, and with tools and educational resources, RCCs found providers to be amenable to managing their SCD patients on HU. More work is still required to ensure that adequate tools and educational opportunities are available to a wider audience of providers, and that providers are appropriately incentivized to increase their comfort with disease management for this rare and complex condition.

Aim 3: Increase Number of Providers Knowledgeable about Treating SCD and Increase the Number of Patients Seen by Knowledgeable SCD Providers

The primary lessons learned for this aim were that many activities and efforts are needed to create a scaffolded approach to create and support networks of providers who are not only willing to treat those with SCD, but appropriately trained, and provide decision support to effectively manage this condition.

Lessons learned around this aim include:

- Network calls (e.g., telemedicine, case presentations) are a critical tool that enable active provider support and mentoring for discussing challenging cases and indications for certain treatments.
- Creation of a list of willing providers who are knowledgeable in treating patients with SCD can be a critical resource for both patients and providers for identifying appropriate sites for care.
- Given time and resource constraints, it remains critically important to look beyond PCPs for educational efforts aimed at the healthcare system. Educational efforts should also target SCD specialists and hospitalists for efforts such as Project ECHO®.
- It is essential to look beyond the healthcare delivery system to the public health system to create sustainable change and to mobilize evidence-based public health approaches.
- All SCD efforts should consider integration and partnerships within the wider blood disorders community to develop synergies between various project activities.
- Workforce development of the next generation of healthcare providers is a critical focus of educational efforts and systems of reward.
- Patients remain critical partners in this work, and patient and family collaboration is key to successfully engaging knowledgeable providers.
- Even when educational opportunities are provided, measuring and linking these activities to increased knowledge and change in practice behaviors can be challenging.
- Project ECHO® is an innovative model and is a well-poised opportunity to create a community of providers who are confident treating SCD, including hematologists.

Collecting data to understand the impact of educational opportunities is a consistent challenge. However, great work has gone on over the past three years to create a wider network of knowledgeable providers treating SCD. The groundwork has been laid to identify effective and innovative practices, create standardized curriculum and standards for educational efforts, and spread educational models to new areas in order to increase the eligible pool of knowledgeable providers while also connecting patients to these providers.

TABLE 3: Project ECHO® Programs

	MIDWEST (STORM	NORTHEAST (SiNERGe)	PACIFIC (PSCRC)	TOTALS
Start date	March 2016	Sept. 2015	Oct. 2016	
Frequency	Monthly	Weekly	Monthly	
SCD Focus	Lifespan	Adult care	Lifespan	
# of sessions held	16	72	7	95 sessions
# of providers participating	15 per clinic; 50 unique	15 per clinic; 135 unique	10 per clinic; 26 unique	10-15 per clinic; 211 unique

RECOMMENDATIONS

While significant progress has been made in both understanding and caring for patients affected by SCD in the past generation, much more remains to be done for this patient population with complex healthcare needs to ensure equitable access to knowledgeable care, proven therapies, tailored forms of treatment, and cost-effective delivery of care. Throughout the three years of the SCDTDP initiative, NICHQ has worked closely with the regional coordinating centers to support their efforts in meeting the program's aims. The accomplishments of the grantees demonstrate that patients living with SCD and their families benefit when providers, community-based organizations and government agencies work collaboratively toward a shared goal of improving the health and quality of life of children and adults who live with SCD.

Enormous opportunities remain to improve the state of SCD care in the United States. In addition to the learned experiences of the grantees, NICHQ regularly engaged with a diverse group of SCD experts, such as the Oversight Steering Committee, with decades of combined experience to develop the series of recommendations outlined below, and found in Section 6 of the complete report, that are focused on future SCD initiatives, healthcare policy, and clinical care.

Recommendations for Future Sickle Cell Initiatives and Programs

It is not a coincidence that patients living with SCD have benefited from better care coordination and access to disease-modifying therapies over the past century. Rather, these advancements are a result of deliberate and direct investments made in programs and partnerships such as the Sickle Cell Disease Treatment Demonstration Program. However, despite the progress made in care that has allowed most children living with SCD to reach adulthood, systemic barriers still impede their ability to receive comprehensive, coordinated care to achieve their optimal health. As more people with SCD are surviving into adulthood, it remains imperative that demonstration programs designed to address and eliminate barriers to SCD care continue. In addition, the following recommendations strengthen the investments already made in SCD initiatives to date.

- Collect coordinated measures across aims and domains of focus, using standardized data definitions and collection methods, to follow and gain understanding of disease progression over time, including young adulthood and beyond
- Enhance the national focus on the use and coordination of sickle cell registries
- Develop standard SCD curricula and metrics of success around the application of the Extension for Community Healthcare Outcomes (ECHO®) model, a practice-guided medical education model, and other telehealth strategies for SCD care to:
    - Increasing providers who are knowledgeable about treating SCD
    - Improving SCD outcomes by ensuring patients have some access to services even if they are not close to a sickle cell program or center
- Maintain improvement science as an integral component of SCD initiatives
- Sustain and provide resources to support interagency coordination and cooperation to amplify the impact and optimize the resources of federal SCD initiatives
- Support regionalization to sustain SCD healthcare systems' capacity building using evidence-based public health practices

Healthcare Policy Recommendations

The complications of managing the care and treatment of SCD can be all-consuming for patients and their families. Increased collaboration between all SCD stakeholders, including patients and families, as well as CBOs, is critical to enhance care coordination and patient and provider education. Involving key stakeholders as true partners in care will lead to better outcomes for patients across their lifespans. To continue to support transformation within the healthcare system, the following recommendations concern policies that when implemented would substantially improve the system of care supporting patients with SCD.

- Improve access to evidence-based SCD care through telehealth, telemedicine, telementoring, and other innovative models, and enhance reimbursement for these services.
- Incorporate standardized quality measures of access and care of patients with SCD into organizational performance measures such as the Bureau of Primary Healthcare quality metrics.
- Make sure that all patients with SCD have consistent access to insurance to help ensure high quality care.
- Ensure adequate reimbursement for care transition that enables seamless and comprehensive care from adolescence through young adulthood and beyond.
- Adjust payment policies, especially for Medicaid recipients, enhancing reimbursement rates to include care coordination services, which would include CHWs, to improve access to community resources, social services, mental healthcare, and clinical services.
- Develop payment systems that support reimbursement of preventive care visits to primary care and specialty care providers as well as social services (e.g., CHWs).
- Ensure there are qualified healthcare professionals providing both pediatric and adult SCD care by creating specific workforce training and student loan forgiveness programs for broader categories of physicians (e.g., hematologists), nurses, nurse practitioners and physician assistants.

Recommendations for Clinical Care Systems

The following clinical recommendations provide further insight into the improvements necessary to ensure that those who treat SCD are adequately trained and supported by data-driven research, as well as ensure a comprehensive system is developed to meet the complex and changing needs of this patient population.

- Implement systems (e.g., electronic health record templates, order sets, tracking and feedback mechanisms) to track and work to increase rates of appropriate screening and preventative interventions (e.g., HU, penicillin prophylaxis, immunizations, and transcranial Doppler screening).
- Address deficiencies in ED care of individuals with SCD experiencing acute pain crises by establishing pain protocols; providing and making widely available pain management plans; and supporting investigation of innovative pain management strategies and routes of administration.
- Implement data systems that enable management of the entire SCD population served through a clinical system or in a geographic area and track key processes and outcomes, including the use of effective therapies (e.g., HU), ED visits, hospitalizations, and readmissions.
- Expand the evidence base related to the use of care plans and other care coordination tools for SCD.
- Design patient and family education regarding use of HU to extend beyond a discussion of benefits and risks to include discussion of patient preferences and strategies for self-management support.
- Require that healthcare systems address both psychosocial and medical needs of individuals with SCD and their families, including routine screening for mental health and social needs.
- Ensure all facilities providing care for individuals with SCD, whether pediatric, adult, or family medicine, incorporate the six core elements of transition as appropriate. This includes having a transition policy; developing a process for tracking and monitoring transition-age youth; assessing and using transition readiness assessments; planning for transition; transferring care; and completing transfers.
- Provide comprehensive care and medical homes where needed for adults with SCD; this is critical for reducing morbidity and mortality rates for this age group.
- Assess current practice patterns for screening of immigrants (including African, Caribbean, Hispanic and Middle Eastern immigrants) for SCD. Develop and/or refine screening processes and link identified individuals to systems of care based on this assessment.
- Use systematic approaches to QI based on data collected and evidence assessment that involve patients and families in both design and implementation.

The 2014-2017 Sickle Cell Disease Treatment Demonstration Program was designed to improve upon a group of measures on a national scale. While significant progress has been made on these measures and in both understanding and caring for patients affected by SCD, much more remains to be done for this high-need patient population to ensure equitable access to knowledgeable care and proven therapies. The accomplishments of the SCDTDP grantees demonstrate that patients living with SCD and their families benefit most when providers, community-based organizations, and government agencies work collaboratively toward a shared aim of improving the health and quality of life of children, adolescents and adults whose lives have been impacted by SCD.

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