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# SICKLE CELL DISEASE TREATMENT DEMONSTRATION PROGRAM

## CONGRESSIONAL REPORT



**OCTOBER 2014**

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The National Coordinating Center for the Sickle Cell Disease Treatment Demonstration Program was supported by the Health Resources and Services Administration's Contract HHS250201000022C.

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## EXECUTIVE SUMMARY

*“When you live with a chronic illness your life revolves around your health. Therefore interacting with the medical staff be it nurses or doctors is an important part of my wellbeing... It is amazing to be able to walk in the emergency room and know what the protocol is because you were a part of implementing it and to know that there are people out there that care and want us to be treated fairly and not suffer in pain... I can also speak my mind and share my ideas and I am being heard. Thank you so much for opening your heart and listening to us. We don't get a lot of people like [TDP team] that don't mind doing the hard work it takes to see changes being made for people who need help advocating for themselves and just want to be heard and listened to.”*

*-SCDTDP patient partner*

Sickle cell disease is a chronic condition disproportionately affecting our country's most vulnerable populations, many of whom experience fragmented, poor quality and often inhumane care.<sup>1,2</sup> Improving the quality of care and overall health of individuals living with sickle cell disease is a critical challenge that requires a multifaceted approach. Multiple stakeholders including patients, family members, primary care providers, specialists, community-based organizations, public health agencies and payers need to work collaboratively to ensure individuals with sickle cell disease have access to a holistic system of care that ultimately leads to optimal health.

From September 2010 to September 2014, NICHQ (the National Institute for Children's Health Quality) served as the National Coordinating Center for the federally-supported Sickle Cell Disease Treatment Demonstration Program (SCDTDP). This report documents the work that NICHQ and its partners, Boston Medical Center, the Sickle Cell Disease Association of America and Family Voices, led during that time. The SCDTDP is administered by the Maternal and Child Health Bureau of the Health Resources and Services Administration (HRSA). In its role as the National Coordinating Center, NICHQ sought to improve the quality of care that individuals with sickle cell disease receive across the lifespan at nine SCDTDP demonstration sites within the United States.

NICHQ and its partners launched this work by convening a panel of leading experts with experience in the clinical science and best practices of treating sickle cell disease, along with current grantee networks and representatives from previous rounds of the program. This panel worked to determine the highest-leverage, evidence-based changes that would result in improving care for this population. The recommendations that came out of this meeting guided the work of the SCDTDP grantees as did ongoing support from project faculty and the Oversight Steering Committee, a collection of individuals who brought unique knowledge, skills, and connections to the project.

Over the four-year project, nine different SCDTDP grantees across the country worked together in the Hemoglobinopathy Learning Collaborative, coming together both virtually and in person to compare data, share results, discuss challenges and solutions, and refine their skills in the methods of improvement science. Since the goals of the Sickle Cell Disease Newborn Screening Program (SCDNBSP)—another HRSA program addressing care for individuals with sick-

le cell disease—were closely aligned with those of the SCDTDP, grantees from the SCDNBSP joined grantees from the SCDTDP in the Hemoglobinopathy Learning Collaborative. Grantees from both programs worked to improve care in many of the same areas, collaborated extensively, used many of the same methods, and collected data on many of the same quality measures. Outcomes from the SCDNBSP sites have been included in this report to Congress where relevant.

Participating grantees used quality improvement methodology to improve care along five core dimensions:

### **1. Ensuring timely, effective, and respectful care in the emergency department**

Excruciating pain crises are a common experience for those with sickle cell disease, and treatment for these pain crises in the emergency department is all too often slow, ineffective, and insensitive. Timely, appropriate and respectful pain management in the emergency department can relieve pain, reduce hospitalizations, and reduce the development of chronic pain symptoms. *Substantial progress was made toward decreasing the time that patients with sickle cell disease must wait to have their pain assessed (69 percent improvement) and decreasing the time between triage and the receipt of first dose of pain medication (29 percent decrease).*

### **2. Ensuring that care is coordinated across primary and specialty providers and services**

Care for individuals with sickle cell disease is often fragmented and uncoordinated, leading to missed appointments, poor medication adherence, and inconsistent provision of recommended components of care such as screenings and immunizations. Well-coordinated care in the context of a medical home, including support for chronic illness self-management, can lead to fewer and less severe complications of sickle cell disease. *The coordination of primary and specialty care was improved in many areas, including the percentage of patients who were evaluated by a hematologist within the past year (increase of 135 percent) and the percentage of patients whose care plans were reviewed during their visit (increase of 170 percent).*

### **3. Improving the follow-up care and counseling for families whose newborns have screened positive for sickle cell disease and trait, and offering screening and counseling to immigrant and adult populations**

Early identification and proper follow up care and counseling is important for individuals with sickle cell disease and sickle cell trait, but newborn screening systems vary greatly state to state and infants with a positive screen can be lost to follow up. Strong screening and follow-up systems have many long-term benefits, including reduced mortality of children with sickle cell disease (from the use of preventative medication) and the ability of those with sickle cell disease and trait to make informed reproductive choices (from genetic counseling). *Grantee networks worked with providers, genetic counselors, families, and state departments of public health to ensure that families received notification of positive screens and that follow up care was provided. They also reached out to immigrant populations and provided free testing and counseling at a variety of community events.*

### **4. Improving the support and education that young adults receive as they transition from pediatric to adult care**

Many individuals with sickle cell disease do not experience a smooth transition from pediatric to adult care. They may not have adequate knowledge or enough practice managing their medications and appointments, and it may be difficult for them to find appropriate adult providers and health care coverage. As a result, mortality rates can be elevated for young adults making this transition. A successful transition program can prepare young adults for this challenging time and help them avoid unnecessary complications of the disease. *Grantee networks developed and tested many tools and resources to use with transitioning young adults. Some grantees saw improvement in process-level measures such as the percentage of adolescents given a transition readiness tool, but overall the program did not see improvement in the outcome measure, the percentage of patients with a written transition plan.*

## 5. Optimizing the use of hydroxyurea, the only therapy for sickle cell disease approved by the Food and Drug Administration

For eligible patients, hydroxyurea can have a tremendous impact on their quality of life by reducing complications of sickle cell disease. However, use of hydroxyurea varies a great deal by provider and by institution, and poor understanding of the drug and its side effects limits its use. *Several grantee networks worked on practice guidelines and educational materials about hydroxyurea.*

Grantees also developed and tested many tools and resources to use with patients with sickle cell disease and sickle cell trait and their families, and conducted an array of educational and community events both for and with these individuals and their families. Finally, the program has led grantees to create and strengthen networks of clinical care sites, federally qualified health centers, and community-based organizations.

## RECOMMENDATIONS

Ten years ago, the Sickle Cell Treatment Act provided funding for projects to demonstrate ways to improve care and outcomes for individuals affected by sickle cell disease. For the past four years, NICHQ and its partners supported these grantees using collaborative learning and quality improvement. This approach entailed regularly collecting data and sharing results and best practices among the grantees, which provided extensive opportunities for learning. This experience forms the basis for our recommendations. The accomplishments of the grantees over the past four years demonstrate the impact that can be realized when patients and families, providers, community-based organizations, and public health and government agencies work collaboratively to improve care for individuals with sickle cell disease. These recommendations were also informed by the challenges that we and the sites encountered, as well as the limits to what we and sites were able to accomplish. Our recommendations address several different levels of action: (1) Recommendations for clinical delivery and public health programs (2) Recommendations for the design or re-design of the Sickle Cell Disease Treatment Demonstration Program and (3) Recommendations for broad health policy.

The system of care for individuals with sickle cell disease should include the main tenets of the patient-centered medical home, and the overall goal of the SCDTDP should be to move beyond simply demonstrating how to improve care for these individuals to spreading these improvements so that all patients with sickle cell disease have access to a system of high quality care. All of the recommendations included in this report are directed towards achieving the aim of the Sickle Cell Treatment Act, which is to improve the health care and outcomes for individuals with sickle cell disease.

### 1. Recommendations for Clinical Delivery and Public Health Programs:

- a. Address deficiencies in emergency department care of individuals with sickle cell disease experiencing acute pain crises by establishing pain protocols, providing and making widely available pain management plans and using more easily administered medications.
- b. Continue to increase access to medical homes and enhance care management and care coordination through the use of care management plans jointly developed by primary care providers, specialists, hospitalists and other inpatient providers with patients and families.
  - i. Expand the evidence base related to the use of care plans and other care coordination tools in sickle cell disease.
- c. Implement systems (e.g., electronic health record templates, order sets, tracking and feedback mechanisms) to increase rates of appropriate screening and preventative interventions (e.g., penicillin prophylaxis, immunizations, hydroxyurea, transcranial Doppler screening).

- d. Ensure education regarding use of hydroxyurea extends beyond a discussion of benefits and risks to include discussion of patient preferences and strategies for self-management support.
- e. Ensure that health care systems address psychosocial needs of individuals with sickle cell disease and their families as well as medical needs.
- f. Ensure all facilities providing care for individuals with sickle cell disease incorporate the six core elements of transition where appropriate, including 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.
- g. Assess current practice patterns for screening of immigrants (including African, Caribbean, Hispanic and Middle Eastern immigrants) for sickle cell disease. Develop and/or refine screening processes and link identified individuals to systems of care based on this assessment.
- h. Involve patients and families in the design and implementation of quality improvement activities.
- i. Involve community-based organizations as partners in programs to improve care for individuals with sickle cell disease across the lifespan.
- j. Implement data systems that enable management of the entire sickle cell disease 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., hydroxyurea), emergency department visits, hospitalizations, and readmissions.
- k. Use systematic approaches to quality improvement, based on data, family engagement, and evidence.

## *2. Recommendations for the Design or Re-design of the SCDTDP:*

- a. We endorse the focus of the new SCDTDP on increasing access to care, increasing the number of providers capable of caring for individuals with sickle cell disease and increasing the use of hydroxyurea, as well as adopting a regional model to spread improvements in care across broader sections of the country.
- b. Resources of the SCDTDP should be aligned with prevalence of sickle cell disease, perhaps initially allocating resources to those regions with higher numbers of affected individuals with a future plan to expand resources to ensure all patients irrespective of geographic location have access to high quality care.
- c. Until all patients with sickle cell disease have access to high quality care, consider implementation of telehealth strategies to ensure patients have some access to services even if they are not close to a sickle cell program or center.
- d. Involve patients and families in program development and program activities to ensure that efforts are responsive to their ongoing needs.
- e. Financial and technical support for data collection should be commensurate with programmatic needs; the current resources are grossly insufficient to collect and report on the necessary data elements.
- f. The Health Resources and Services Administration should align funding cycles of the National Coordinating Center and program grantees to ensure similar start and end dates.
- g. The Health Resources and Services Administration should require the National Coordinating Center and program grantees to adopt a shared measurement strategy and data collection system.
- h. Improvement science should remain an integral component of the SCDTDP.
- i. Interagency coordination and cooperation could amplify the impact and optimize the resources of the SCDTDP. This can occur across the bureaus of the Health Resources and Services Administration, e.g., through engagement with the Bureau of Primary Health Care, as well as across other agencies within the

Department of Health and Human Services and beyond. These other agencies include the Centers for Disease Control and Prevention, the Centers for Medicare and Medicaid Services (including its Center for Medicare and Medicaid Innovation), the Agency for Healthcare Research and Quality, the National Institutes of Health, the Office of Minority Health and others.

j. The work of the SCDTDP and the SCDNBSP should be aligned. Collaboration between grantees and the coordinating centers will maximize resources and impact while limiting duplication.

### 3. Recommendations for Health Policy:

The health care needs of this population should be addressed through broadly implemented health policies rather than relatively small demonstration programs. Specific policy options might include:

- a. New payment models that ensure that all patients with sickle cell disease have consistent insurance access to high quality care that is linked to a quality performance reporting and improvement system (e.g., categorical eligibility for Medicare for patients with sickle cell disease, analogous to individuals with end-stage renal disease, regardless of age).
- b. Adjusting Medicaid payment policies and enhancing reimbursement rates to include care coordination services for this population, as was recently implemented for Medicare.
- c. The Center for Medicare and Medicaid Services should develop risk-based capitation strategies for sickle cell disease.
- d. Consider specific reporting on readmissions for sickle cell disease in hospitals; this might be paired with financial incentives with appropriate adjustment for severity of illness and other indicators of risk.
- e. Adopt recently developed performance measures for sickle cell disease into insurance programs (Medicaid, Children's Health Insurance Program, Medicare) across the lifespan. Incorporate these measures, or a subset of them, in the Bureau of Primary Health Care quality performance measures.
- f. Specific workforce training programs for health care professionals interested in caring for individuals with sickle cell disease. Provide enhanced compensation and potential loan forgiveness programs for hematologist/oncologists committing to at least a minimum number of patients with sickle cell disease or proportion of their practice devoted to patients with sickle cell disease.
- g. Incorporate sickle cell disease-specific requirements in federal regulations for meaningful use.
- h. More broadly, assure that all federally supported health care programs (e.g., federally qualified health centers, Department of Defense and Veteran's Administration programs) apply the clinical recommendations noted above.

The current Sickle Cell Disease Treatment Demonstration Program has made great strides in improving the quality of care for individuals with sickle cell disease. Grantees were able to apply improvement science methods to make improvements in several processes of care that positively affect patients. These improvements include more timely and compassionate care in emergency departments, increased access to providers, and more reliable provision of recommended screenings and therapies. The encouraging results and work described in this report have provided a number of important lessons:

- I. Targeted strategies implemented using a disciplined change approach can lead to significant improvements in the quality and timeliness of treatment in the emergency department and enhance patient experience of care.



2. Use of patient navigators, community health workers, community-based organizations and patient self-management tools can improve access, coordination and integration of services for patients with sickle cell disease.
3. An early and comprehensive approach to transition, combined with self-management support can help mitigate the many challenges that individuals with sickle cell disease face during this vulnerable time.
4. Multilevel interventions targeted at the patient, family, provider and system can increase hydroxyurea use.
5. Opportunity still exists to improve follow up care after screening to ensure patients are enrolled in comprehensive care. Further work is needed to identify the appropriate processes for screening immigrant populations for sickle cell disease.
6. A shared and coordinated measurement strategy across grantee networks can enhance the program's ability to measure improvements in key process and outcomes related to sickle cell care. Coupling the measurement with a systematic approach to improvement results in better care and will ultimately lead to better outcomes.

The current Sickle Cell Disease Treatment Demonstration Program has demonstrated that better care for individuals with sickle cell disease is possible. This report has synthesized what can and should be done to improve care and provided recommendations for how these improvements can be implemented. The recommendations regarding modifications to the Sickle Cell Disease Treatment Demonstration Program provide an opportunity for how this program can enable even greater learning and have greater impact on the populations directly touched by grantee programs. Yet what are most needed are mechanisms to move these lessons into widespread practice and to address barriers (such as an insufficient provider workforce) beyond the scope of the currently designed program.

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## ACKNOWLEDGEMENTS

NICHQ is grateful for the tremendous dedication of time and outstanding work of the authors of this report, as well as the grantee networks whose work this report represents. Throughout the development of this report, NICHQ sought input from representatives from each grantee network as well as experts including project faculty and the Oversight Steering Committee. The synthesis of the data and recommendations presented in this report do not necessarily represent the views of individual reviewers.

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THE NATIONAL COORDINATING CENTER FOR THE SICKLE CELL DISEASE TREATMENT DEMONSTRATION PROGRAM WAS SUPPORTED BY THE HEALTH RESOURCES AND SERVICES ADMINISTRATION'S CONTRACT HHS250201000022C.

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## INTRODUCTION

Sickle cell disease is a debilitating disease affecting between 70,000 and 100,000 Americans, predominantly those of African descent. The disease is characterized by unpredictable periods of extreme pain, caused when sickled red blood cells are unable to move freely through blood vessels. In addition to pain, these changes at the cellular level can also lead to organ damage, stroke and even death among some individuals with sickle cell disease.

Recent developments in sickle cell healthcare have changed the face of this disease. Just a generation ago, most patients were not expected to survive into adulthood. Advances in treatment and interventions have helped many with sickle cell disease manage their disease successfully and live fuller, longer lives. However, many barriers at the family, provider and health system level contribute to the current state of fragmented and costly care. These challenges have been recognized by those in Congress over the past four decades, resulting in legislative and federal initiatives dedicated to the advancement of sickle cell healthcare. These federal initiatives, including the Sickle Cell Disease Treatment Demonstration Program (SCDTDP), which is the main focus of this report, played a significant role in advancing sickle cell healthcare to see the successes that we see today.

This report to Congress synthesizes the results of four years of the SCDTDP, one of two federal programs administered by the Health Resources and Services Administration (HRSA) of the Department of Health and Human Services that aim to improve the quality of care that individuals with sickle cell disease receive in the United States. The SCDTDP consists of multiple demonstration sites across the country as well as a National Coordinating Center.

From September 2010 to September 2014, NICHQ (the National Institute for Children's Health Quality) served as the National Coordinating Center for this important program. This report includes:

- **A brief overview of sickle cell disease and gaps in care currently experienced by those with the disease;**
- **Background on the approach and methods that were used during the past four years by the demonstration sites and the National Coordinating Center;**
- **An analysis of qualitative and quantitative outcomes that were seen at particular sites and in the aggregate during this time period;**
- **A conceptual framework for interpreting these results;**
- **A synthesis of lessons learned during the past four years; and**
- **Recommendations for the future of the program.**

Appendices to this report provide more detail regarding methods, measurement and clinical interventions. In particular, the changes tested by grantees that are listed in the Results and Impact portion of Section 2 are detailed more fully in the SCDTDP Model Protocol (Appendix 1), along with the associated tools and resources used by grantees.

From 2011 to 2014 the aims and approach employed by the SCDTDP and the Sickle Cell Disease Newborn Screening Program (SCDNBSP)—another HRSA program addressing care for individuals with sickle cell disease—were closely aligned. As the National Coordinating and Evaluation Center for the SCDNBSP, NICHQ worked closely with HRSA to design a process where sites receiving grants under each program worked to improve care in many of the same areas, collaborated extensively, used many of the same methods, and collected data on many of the same quality measures. In the interest of reporting data that is as robust as possible and advancing knowledge about the improvement of



care for those with sickle cell disease, outcomes from the SCDNBSP sites have been included in this report to Congress where relevant, even though SCDNBSP is a separately funded program whose aims are not identical to those of the SCDTDP.

Though this report is intended to disseminate knowledge to a wide audience, it includes some terminology of a medical or technical nature. A glossary of terms is included to help readers who may not be familiar with these specialized domains (see Appendix 2); the words included are underlined within the text.

## BACKGROUND AND SIGNIFICANCE

### Overview of Sickle Cell Disease

Sickle cell disease is a group of inherited red blood cell disorders that affects between 70,000 and 100,000 people in the United States (US).<sup>1,2,3</sup> Sickle cell disease is caused by inheriting two altered genes, one from each parent, that result in abnormal forms of hemoglobin, a protein that carries oxygen to tissues throughout the body.<sup>2,3</sup> An additional two million Americans have sickle cell trait, meaning that they have inherited one gene for the abnormal hemoglobin (sickle hemoglobin). Individuals with sickle cell trait are genetic carriers for the disease. In the US, most cases of sickle cell disease occur among people of African ancestry (1 out of every 360 African American births). The frequency among Hispanics in the US is generally lower than African Americans, affecting 1 out of every 16,300 births.<sup>3,4</sup> Sickle cell disease is also found among individuals from Caribbean, Mediterranean, Middle Eastern or Indian backgrounds. Recent state-level data suggest that the frequency of sickle cell disease in the US varies state to state and is influenced by the number of foreign-born parents from countries with a high incidence of the disease.<sup>3</sup>

Individuals with sickle cell disease experience significant health problems such as chronic anemia that reduces stamina, frequent episodes of extreme pain, pulmonary complications (e.g., acute chest syndrome, which can be life-threatening), and stroke. Persons living with this disease also experience acute and chronic complications related to anemia, chronic organ damage, infection and psychosocial issues, all of which can lead to a greatly diminished quality of life. Life expectancy is clearly improving with current prevention and treatment interventions, but is still shortened. Early diagnosis of sickle cell disease is critical so that children who have the disease can receive proper interventions, such as daily prophylactic penicillin, early management of fever, and immunizations. Newborn screening for sickle cell disease followed by parental health education, enrollment in comprehensive care and initiation of established therapies prevents complications and early death. Over time, proper health management, including ongoing health care visits, screenings and appropriate care across the lifespan, are essential for preventing or minimizing many sickle cell related complications so that the individual can have a more productive and higher quality life.

### Disparities in Care and Need for Improvement

Unfortunately, significant variation exists in the quality of care and health outcomes for individuals living with sickle cell disease across care sites and institutions in the United States.<sup>5,6,7</sup> Patients fail to receive well-established therapies and recommended screenings for this condition and its complications.<sup>8,9,10,11,12</sup> Care for affected individuals is often fragmented and spans multiple institutions, resulting in many persons not having a medical home that coordinates their care.<sup>13</sup> The number of hematology specialists with expertise in the care of persons with sickle cell disease has decreased in recent years, resulting in a dearth of specialty providers, particularly those serving adult patients.<sup>14,15,16</sup> Specifically, care for adults is often fragmented because they are seen in emergency rooms for acute problems, are cared for by hospitalists when hospitalization is required, and lack a primary physician coordinating their care. This leads to reduced quality of care and increased utilization of resources. Geographic, economic, and cultural barriers also limit access to the care that is required to prevent morbidity and mortality.

New models of care that effectively integrate primary care, subspecialty care, and social service supports are necessary to improve care for individuals with sickle cell disease.<sup>17</sup> Moreover, ongoing strategies to improve patient access to and knowledge about effective treatments are essential to improve outcomes for individuals with the disease. As with all chronic diseases, particularly ones that involve ongoing pain and acute life-threatening complications, support

for patient self-management is also critical. In this context, integrated networks of primary care providers, emergency departments, inpatient services, community health centers, community organizations, hematology providers and individuals with sickle cell disease can play a pivotal role in ensuring persons with sickle cell disease receive high quality, coordinated, culturally appropriate, and patient-centered care.

### **Federal Sickle Cell Disease Legislation**

During the past four decades, several federal initiatives addressed screening for and care of individuals with sickle cell disease. The Sickle Cell Anemia Control Act was signed into law by President Nixon in 1972, and provided funding for voluntary screening, development and dissemination of educational materials, and research in the diagnosis and treatment of the disease.<sup>18</sup> The research that stemmed from this funding led to transformative improvements in care and a significant increase in the life expectancy for individuals with the disease.<sup>19</sup> The success of new therapies, such as prophylactic penicillin for young children (discussed further below), led to a national recommendation for universal newborn screening for the disease in 1987.<sup>20,21</sup>

In 2002, Congress appropriated funds for the creation of the SCDNBSP “to enhance the sickle cell disease newborn screening program and its locally based outreach and counseling efforts.”<sup>22</sup> The SCDNBSP seeks 1) to improve the follow up of individuals detected through newborn screening with sickle cell disease, sickle cell trait and other hemoglobinopathies; 2) to ensure that all individuals identified receive the highest quality of health care and support through their lifespan; and 3) to ensure that all individuals identified receive appropriate education and counseling.

In 2004, Congress enacted and President George W. Bush 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, which expanded the federal government’s support for improving the care that individuals with the disease receive. The resulting program, known as the Sickle Cell Disease Treatment Demonstration Program, seeks to enhance treatment through funding programs that might provide coordination of service delivery, genetic counseling and testing, bundling of technical services, training of health professionals, and other related efforts.

### **Overview of the Sickle Cell Disease Treatment Demonstration Program**

The SCDTDP is administered by the Maternal and Child Health Bureau of HRSA and provides grants to federally qualified and other nonprofit health care centers. These grants have helped to establish local networks that work with comprehensive sickle cell disease centers and community-based support organizations to provide coordinated, comprehensive, culturally competent, and family-centered care to families of individuals with sickle cell disease and sickle cell trait. The SCDTDP seeks to improve coordination and service delivery for individuals living with sickle cell disease, improve access to services, and improve and expand patient and provider education.

Under the authorizing legislation, a National Coordinating Center was also 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 treatment of sickle cell disease; and, (4) prepare a final report on the efficacy of the demonstration program based on evaluation findings.

From 2010-2014, the period covered in this report, nine demonstration sites received funding from HRSA under the SCDTDP. NICHQ and its partners, Boston Medical Center, the Sickle Cell Disease Association of America and Family Voices, were awarded the contract for the National Coordinating Center in 2010.

## SICKLE CELL DISEASE TREATMENT DEMONSTRATION PROGRAM PARTICIPANTS 2010-2014

### Health Resources and Services Administration

HRSA, an agency of the U.S. Department of Health and Human Services, is the primary federal agency for improving access to health care. The Genetic Services Branch of the Division of Services for Children with Special Health Care Needs within the Maternal and Child Health Bureau of HRSA administers the SCDTDP. The Maternal and Child Health Bureau worked closely and cooperatively with the grantee networks (also referred to as grantees throughout this report) and with NICHQ to develop the structure, methods and content of the program, to monitor progress over time, and to plan specific events and tasks. The HRSA Project Officer for the program met with NICHQ on a monthly basis and senior leaders of the Maternal and Child Health Bureau met with NICHQ on a quarterly basis to ensure ongoing communication about program activities. At the beginning of the project, NICHQ developed and the HRSA Project Officer approved a master plan for NICHQ's work as the National Coordinating Center.

### National Coordinating Center

NICHQ is an independent, nonprofit organization that has worked for more than a decade to improve children's health by helping organizations and professionals who share this mission (typically, healthcare professionals and delivery organizations, foundations, government agencies, and community organizations) make breakthrough improvements so children and families live healthier lives. Since its founding in 1999, NICHQ has conducted dozens of improvement projects using a variety of methods and approaches and focusing on a wide range of diseases and conditions.

NICHQ used its experience and expertise in improvement science to bring a clearer focus on quality improvement to SCDTDP, making ongoing collaborative quality improvement a central vehicle for sharing and disseminating best practices among the program's grantees to accelerate improvement. As the National Coordinating Center, NICHQ was charged with managing all components of the program, including developing a measurement and evaluation strategy, collecting and analyzing data, providing training in quality improvement methods, providing technical assistance to grantees, increasing access to relevant educational materials, and disseminating findings and best practices.

NICHQ worked with three key partners to help achieve the collective aims of the National Coordinating Center:

- Boston Medical Center brought expertise in developing quality measures, data analysis, and information technology applied to quality improvement. Boston Medical Center's main function was to support the program's use of data.
- The Sickle Cell Disease Association of America provided content expertise, worked with Family Voices and other subcontractors to enhance the capacity of community members to advocate for better healthcare and service for individuals with sickle cell disease and sickle cell trait, and managed the on-site logistics for program-related meetings.
- Family Voices provided expertise in engaging families with children who have special health care needs. As a partner in this project, Family Voices helped to build the Sickle Cell Disease Association of America's capacity to work with consumers. Family Voices also worked directly with consumer representatives to ensure that this important perspective was well represented.

As mentioned above, NICHQ worked with HRSA to design a process where grantees of the SCDTDP and the SCD-NBSP worked to improve care in many of the same areas, collaborated extensively, used many of the same methods, and collected data on many of the same quality measures from 2011 to 2014. NICHQ referred to this collaboration as the Working to Improve Sickle Cell Healthcare (WISCH) project.

## Grantee Networks

### Sickle Cell Disease Treatment Demonstration Program

During 2010-2014, the Maternal and Child Health Bureau of HRSA awarded cooperative agreements to nine grantees in California, Colorado, Illinois, Maryland, Missouri, New Jersey, Ohio, Pennsylvania and Tennessee under the SCDTDP (see Table 1). While not paying for direct clinical services, these grants supported additional personnel and systems that enabled services to be more coordinated, comprehensive, culturally competent and family-centered. Grantees were geographically distributed and consisted of federally qualified health centers, nonprofit hospitals or clinics and/or university clinics that provide primary care and specialized sickle cell care, and community-based support organizations or non-profit entities that work with individuals with sickle cell disease and their families within the defined area. Grantees also partnered with individuals with the disease in order to bring the consumer perspective to their improvement efforts.

### Sickle Cell Disease Newborn Screening Program

From 2011-2015, the Maternal and Child Health Bureau of HRSA also funded six grantees in Illinois, Massachusetts, New York, Ohio, Pennsylvania, and Tennessee under the SCDNBSP (see Table 2). As with SCDTDP grantees, SCDNBSP grantees include federally qualified community health centers and other primary care sites, comprehensive sickle cell treatment centers and community-based organizations. The SCDNBSP grantees also partnered with individuals with sickle cell disease and their families. Unique to the SCDNBSP, these grantees also worked in close partnership with their state public health newborn screening programs. Although the SCDNBSP uniquely focused on screening, similar to the SCDTDP sites, these grantees worked to improve follow-up care, develop best practices in care coordination, improve access to medical homes for adults and children, and optimize transition of care.

**TABLE 1:** SCDTDP grantee sites 2010-2014

State	Primary grantee	Primary grantee type	HRSA region
California	Children's Hospital & Research Center <i>Oakland, CA</i>	Hospital	Region 9
Colorado	University of Colorado Denver <i>Denver, CO</i>	University hospital	Region 8
Illinois	Christian Community Health Center <i>Chicago, IL</i>	Federally qualified health center	Region 5
Maryland	Johns Hopkins University Medical Center <i>Baltimore, MD</i>	University hospital	Region 3
Missouri	Washington University School of Medicine <i>St. Louis, MO</i>	University hospital	Region 7
New Jersey	Newark Beth Israel Medical Center <i>Newark, NJ</i>	Hospital	Region 3
Ohio	University of Cincinnati College of Medicine <i>Cincinnati, OH</i>	University hospital	Region 5
Pennsylvania	Primary Care Health Services <i>Pittsburgh, PA</i>	Federally qualified health center	Region 3
Tennessee	St. Jude Children's Research Hospital <i>Memphis, TN</i>	Hospital	Region 4

**TABLE 2:** SCDNBSP grantee sites 2011-2015

State	Primary grantee	Primary grantee type	HRSA region
Illinois	Sickle Cell Disease Association of Illinois <i>Chicago, IL</i>	Community Based Organization	Region 5
Ohio	Cincinnati Children's Hospital Medical Center <i>Cincinnati, OH</i>	Hospital	Region 5
Massachusetts	Boston Medical Center <i>Boston, MA</i>	University Hospital	Region 1
New York	Bronx-Lebanon Hospital Center <i>Bronx, NY</i>	Hospital	Region 2
Pennsylvania	Children's Hospital of Pittsburgh <i>Pittsburgh, PA</i>	University Hospital	Region 3
Tennessee	Vanderbilt University Medical Center <i>Nashville, TN</i>	University Hospital	Region 4

## NICHQ'S APPROACH AND RESPONSIBILITIES AS THE NATIONAL COORDINATING CENTER

### The Hemoglobinopathy Learning Collaborative

As mentioned above, the specific tasks that NICHQ as the National Coordinating Center was required to fulfill were 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.

Because the SCDTDP overall seeks to close the gap between what is known about optimal care for people with sickle cell disease and what is commonly done, NICHQ chose to fulfill its role as the National Coordinating Center by applying the principles and tools of improvement science (quality improvement) to care for individuals with sickle cell disease. A widely used approach to quality improvement in health care is a "Breakthrough Series™ Learning Collaborative," described in detail below. NICHQ used this approach, substantially redesigning the Hemoglobinopathy Learning Collaborative compared to the approach used prior to 2010. NICHQ encouraged the development of shared goals across multiple networks and created opportunities and vehicles for frequent interaction and learning from data. The structure of the Hemoglobinopathy Learning Collaborative provided a way for NICHQ to coach networks as they implemented projects focused on subjects such as transition from pediatric to adult care and facilitating effective coordination between comprehensive care centers and primary care. Technical assistance was provided to grantees individually and collectively through calls, coaching, web resources, live meetings and on-site visits, as needed. Through creating a forum that incorporates improvement science and collaborative learning, the Hemoglobinopathy Learning Collaborative was designed to accelerate the individual efforts of grantees.

Grantees in the SCDNBSP also participated in the Hemoglobinopathy Learning Collaborative, worked to improve care in many of the same areas, collaborated extensively, used the same methods, and collected data on the same performance measures.

## Key Project Tasks and Responsibilities of the National Coordinating Center

### *Collect, Coordinate, Monitor, and Report on Best Practices and Findings Regarding the Activities of the Demonstration Program*

A vital component for any improvement effort is a data collection strategy to monitor progress in meeting goals, drive improvement and share information (successes and challenges) related to improvement efforts. Grantees collected two sets of data for this project: (1) process-level data derived from the medical record and collected on a monthly basis to monitor and drive improvements in care intended to affect outcomes and to inform collaborative activities, and (2) client survey data collected annually to assess patient health status, health care utilization and health-related quality of life. The client survey data primarily assessed health outcomes. The medical record data primarily focused on processes of care. Detailed information on the client survey data collection strategy and the development of the quality improvement measures and data collection system can be found in Appendix 3 of this report. In addition to the data that grantees submitted to NICHQ, several other mechanisms were used to gather information from grantees. These included monthly collaborative wide calls, monthly calls with individual grantees, site visits and reviewing grantees' reports to HRSA. These activities are described in more detail below.

### *Development of a Model Protocol*

At the start of the project, NICHQ convened a panel of experts on sickle cell disease to inform a set of measures and share change ideas that could be used to track and improve quality of care delivered to patients with sickle cell disease (See Table 1, Appendix 4 for the list of experts). The Hemoglobinopathy Learning Collaborative provided an effective and efficient mechanism to collect, coordinate, monitor and distribute data and findings from grantees, adding important experience to this set of measures and change ideas. NICHQ has used this information to identify promising strategies with respect to the prevention and treatment of sickle cell disease. These collective strategies across several dimensions of care are compiled into one document, the SCDTDP Model Protocol (See Appendix 1).

### *Improving Access to Educational Materials*

NICHQ has collected and organized educational materials that have been developed and used by grantees with individuals with sickle cell disease or sickle cell trait, their caregivers, and health professionals. Resources were shared widely within the grantees via posting on the project website or electronic distribution through the project listserv. NICHQ also created an online resource library so that the educational materials can be disseminated more broadly. These resources have been included in the SCDTDP Model Protocol, where relevant.

### *Final Report to Congress*

This report will serve to summarize and disseminate the important knowledge gained through the demonstration program, and thereby inform future work in this area. It addresses the overall impact of the program, including patient and family experiences, specific results of the improvement activities and client survey data collection, and lessons learned over the course of the program.

The two sections that follow present the results of the work that the grantees undertook during the course of the Collaborative, the data for which were collected using two different data streams. The quality improvement work is described in detail in Section 2, Improving Quality of Care for Individuals with Sickle Cell Disease, including rich quantitative and qualitative data demonstrating the breadth and depth of activities undertaken by grantees, from improving the experience of care in the emergency department, to the ways in which the grantees engaged individuals with sickle cell disease and their families to inform their improvement work, to increasing provider and patient education and awareness about disease-modifying therapies such as hydroxyurea. Section 3, Assessing Healthcare Use

and Health-Related Quality of Life, explains and interprets the quantitative data collected through survey instruments collected annually during the project, which provide a longer-term overview of the impact of the work the grantees. Section 4, Considerations for Understanding Data on Health Services and Hydroxyurea Use, offers a synthesis of the results from the two data streams covered in the preceding sections. Finally, we offer overall lessons learned and recommendations for the future of the SCDTDP in the final section, which summarizes the learnings from the grantees and the National Coordinating Center over the course of the program, and in turn uses these to make suggestions for the work that still needs to be done to continue to improve care for individuals with sickle cell disease and their families.

## IMPROVING QUALITY OF CARE FOR INDIVIDUALS WITH SICKLE CELL DISEASE

### METHODS

#### Organizing Frameworks

NICHQ's approaches, methods and expertise are based on the science of quality improvement. In particular, NICHQ utilizes two complementary frameworks, the Breakthrough Series Learning Collaborative and the Model for Improvement.

#### *The Breakthrough Series Learning Collaborative*

A Breakthrough Series Learning Collaborative is a vehicle for refining and spreading changes demonstrated effective for improving care and outcomes for defined populations. Developed by the Institute for Healthcare Improvement, this approach to collaborative learning has been adapted and used by NICHQ over the past 15 years. In this model, teams gather regularly via webinars and face-to-face learning sessions to assess current performance and progress towards project goals. With the assistance of faculty experts in the science of improvement and clinical practice, participating teams share ideas and strategies, learn about essential improvement techniques and prioritize possible actions in terms of impact and feasibility. This process has been tested and refined extensively,<sup>23, 24, 25</sup> and NICHQ has applied it to accelerate change and improvement in hundreds of organizations and systems ranging from individual clinical practices to state governmental agencies and state-wide systems of care. The Breakthrough Series model provides a structured sequence for planning learning sessions, action periods and collaborative calls. The details of each of these collaborative activities are described below. Please see Appendix 4 for an illustration of how the Breakthrough Series model was applied to the SCDTDP, as well as a driver diagram representing the project's theory of change.

#### *The Model for Improvement*

The Model for Improvement provides a framework for improvement activities. The model identifies four key elements of successful process improvement: specific and measurable aims, measures of improvement that are tracked over time, key changes that result in desired improvement, and a series of testing cycles during which teams learn how to apply key changes in their own organizations. The Model for Improvement uses a structured process whereby teams build on small tests of change while measuring and reporting on the impact of those changes on key process and outcome measures. Ideas for affecting changes in the system are evaluated serially using a practical adaptation of the experimental paradigm, the Plan-Do-Study-Act cycle. These cycles test changes, initially on a very small scale, in order to quickly identify promising ideas. Then the results encourage testers to adapt and develop these ideas into robust, reliable standard processes. As a collaborative moves forward, teams gradually begin to embed planning, measurement and testing into their routine work.

#### Structure of the Hemoglobinopathy Learning Collaborative

The Hemoglobinopathy Learning Collaborative was based on the Breakthrough Series model but spanned a longer timeline and involved several additional components added to meet the specific needs of the project. The core components of the Hemoglobinopathy Learning Collaborative are described below.



### *Learning Sessions and Action Periods*

During learning sessions, grantees came together to learn promising practices and a specific approach to making organizational changes using the Model for Improvement framework. Grantees spent a substantial proportion of their time in these sessions planning changes and analyzing their progress with input from colleagues and experts, as well as sharing their results with one another. They developed strategies to overcome barriers to change and planned for further spread of the changes. The periods between learning sessions, called action periods, were at the heart of the Hemoglobinopathy Learning Collaborative's success. During these periods, grantees planned and executed Plan-Do-Study-Act cycles, learning over time which changes were most effective at each site. Although the typical Breakthrough Series Learning Collaborative entails three face-to-face learning sessions over an 18 month period, the Hemoglobinopathy Learning Collaborative held six learning sessions, four in person and two virtual, and six action periods over three years.

### *Monthly Action Period Calls*

Each month, members of each grantee came together for a virtual meeting, known as an action period call. These calls provided an opportunity for grantees to share their work with one another; to get help from improvement advisors; and for faculty, partners and members of grantee networks to present clinical content and lead discussions. Action period calls have focused on topics such as engaging consumer partners, finding and using educational materials, the work being done by the affinity groups (described below), engaging leadership, and interpreting data. Often, action period calls were devoted entirely to grantees sharing their current work. On a periodic basis, additional calls were held to cover topics of interest, such as evaluation plans, logic models, and engaging leadership.

### *Affinity Groups*

Midway through the project NICHQ launched topic-specific working groups, or affinity groups, in response to grantees' interest in doing focused work within certain content areas. The five active affinity groups represented the main areas of focus for grantees: transition of care, medical home/care coordination, self-management, newborn screening and follow up, and acute care. Affinity groups provided grantees the opportunity to learn directly from each other about their work and to generate change ideas together in real time. These groups met on a monthly basis and were led by grantee representatives, while NICHQ staff coordinated the activities and faculty members advised the groups.

### *Workgroup for Sickle Cell Disease Partners*

Each grantee worked closely with individuals with sickle cell disease and their families, who were called sickle cell disease partners. Sickle cell disease partners were vital team members who helped to inform and carry out the work within each of the networks. This workgroup brought together patient and family representatives from most grantee networks on a monthly basis. The goal of this group was to empower partners to have a strong voice and ensure that the patient and family perspective permeated throughout the program activities. These calls allowed partners to discuss tools and resources and to share knowledge about sickle cell disease, sickle cell trait, and quality improvement. Two faculty members, Sherry Richardson of Family Voices and Efa Ahmed-Williams, the Patient Chair for the Collaborative, facilitated this group, with NICHQ staff coordinating the activities on a monthly basis.

### *Individual Technical Assistance*

NICHQ offered individual technical assistance through monthly check in meetings between grantee leads and NICHQ project managers, during which members of grantees had the opportunity to discuss their data, get feedback on their work, and ask questions. Experts in sickle cell disease and quality improvement were available to each grantee through this forum a couple of times each year. Regular office hours were also available as needed with faculty and staff.

### *Site Visits*

NICHQ conducted site visits with several of the grantees over the course of the program. The purpose of the visits was to learn more about the work of the grantees and provide technical assistance. Site visits were made to the Ohio, Missouri, Maryland, Illinois and Pennsylvania SCDTDP grantees.

## Quality Improvement Measure Development

The quality improvement data collection strategy incorporated measures that were developed through a rigorous evidence-based process. Quality measures were identified and narrowed from a previously published set of pediatric sickle cell disease measures.<sup>26</sup> The scope of the measurement set was expanded to include adult aspects of sickle cell disease care. A panel including external experts, patients and parents and several grantee representatives rated all of these measures for validity and feasibility. The final set of measures represents key care processes and outcomes in the care for individuals with this disease. The grantees, as well as the collaborative faculty and leadership, relied on these measures to assess the quality of care at the grantee sites and especially the extent to which grantee improvement activities resulted in improvements in care and outcomes. The full measure set can be found in Appendix 5, and a list of experts who contributed to the development of the measure set can be found in Appendix 4.

### *Technology Support and Information Sharing for the Hemoglobinopathy Learning Collaborative*

The targeted performance measures for the project required capture of data from multiple settings (emergency department, ambulatory care, and community-based organizations) and from multiple stakeholders (patients, parents, clinicians, and community participants). To capture these data, a flexible measurement system that provided an online data capture tool and a patient survey tool was needed. The project also required a system that was easy to use, web-based, and that did not involve extensive software development. All of these requirements were met by the Research Electronic Data Capture (REDCap) system developed at Vanderbilt University and recently implemented at Boston University (and many other sites across the United States). The system created for the project, the Sickle Cell REDCap System, has a number of features designed to facilitate the needs of the SCDTDP; specifically, data capture, measure generation, and information-sharing (Table 3).

Collaborative quality improvement requires the ability to monitor others' performance data as well as one's own. NICHQ developed a mechanism for grantees to share and view aggregate data through the use of an innovative Web portal designed for collaborative quality improvement activities named the Improvement Lab (ILab). The ILab facilitated communication and resource sharing and allowed grantees to view data across the Hemoglobinopathy Learning Collaborative. Improvement advisors assessed these data and provided constructive feedback to grantees to drive the testing and implementation of best practices.

**TABLE 3:** Features of the information technology support for the Hemoglobinopathy Learning Collaborative

<b>Sickle Cell REDCap System:</b>
<ul style="list-style-type: none"><li>• A web-based, secure data capture system</li><li>• De-identified data used to protect privacy</li><li>• Each site maintains a local code list linking the Sickle Cell REDCap System participant identification number with a local medical record number, name, address, and phone number</li><li>• Limited set of patient demographic data (i.e., state of residence, year of birth, gender, and sickle cell genotype) stored in a patient profile</li><li>• Emergency department and outpatient visits sampled and reviewed to capture required data for quality measurement</li></ul>
<b>Measure Up:</b>
<ul style="list-style-type: none"><li>• Sickle Cell REDCap System data automatically extracted for analysis</li><li>• Quality measures used are based on expert recommendations</li><li>• Library of measure formulas and configurable reference data support development and generation of a wide variety of quality measures without the need for extensive analytic programming</li><li>• Aggregate measure data is shared with the Improvement Lab (see below) via an automated web-service</li></ul>
<b>Improvement Lab:</b>
<ul style="list-style-type: none"><li>• A web-based application that allows geographically dispersed teams and users to collaborate by sharing performance data and improvement activities</li><li>• Participants can compare their performance for different measures within their team and for specific measures between teams via on-line run charts</li><li>• Successful (and unsuccessful) changes are shared via annotations directly on each teams run charts</li></ul>

## Quality Improvement Data Collection and Analysis

Regular data collection is a central activity of all improvement projects. The data collection involved in a quality improvement collaborative is focused on identifying measures that are sensitive enough to determine if the changes a team makes truly lead to improvement. Data are tracked over time and reviewed by teams and collaborative faculty. These measures allow teams to analyze data and tell whether improvement is achieved in organizational processes as well as improved care outcomes. For example, SCDTDP grantees focusing on improving care in acute care settings assessed whether implementing pain management protocols reduced the average time until administration of the first pain medication.

Grantees collected quality improvement data at both quarterly and monthly intervals. These data were collected through medical chart review, observation, or through reports pulled from electronic health records. Grantees collected monthly data from a sample of approximately 20 patients with sickle cell disease who were seen in their network that month; the selection of 20 patients per month has frequently been used for improvement work and aided grantees in expediting their improvement efforts while minimizing the data collection burden. Quarterly data were collected from a sample of 80 percent of a grantee's population or up to 100 patients. Each month grantees entered de-identified data directly into REDCap or uploaded spreadsheets into NICHQ's ILab. (Please see Appendix 3 for more detail on the ways in which these data were displayed and interpreted.)

## Program Evaluation

Qualitative and quantitative data were also collected for both formative evaluation (understanding program implementation) and summative evaluation (understanding program outcomes). NICHQ's goal was to create an ongoing system of learning on the network level, including many network stakeholders and spanning all major grantee activities. NICHQ incorporated interim evaluation data to adapt methods and processes, inform daily project work, and refine long term strategic planning. Formative evaluation questions included the strengths and opportunities for improvement with regard to project activities, technical assistance, trainings, and technological support. Summative evaluation questions addressed the SCDTDP's overall success at meeting its aims and objectives. Evaluation activities included key stakeholder interviews, focus groups, surveys, and assessment of engagement levels.

A key focus of the evaluation was to understand how contextual factors at multiple levels (e.g., institution, grantee) influenced the success of the SCDTDP grantees. There is increasing recognition of the importance of knowing how contextual factors influence quality improvement teams differently so that one can better understand the results seen in a collaborative. NICHQ's evaluators developed a contextual factors framework drawing from literature on evaluating contextual factors and quality improvement<sup>27</sup> and adapted based on input from project staff and SCDTDP grantees. The final framework is included in Appendix 4 as a graphical illustration of the various levels (and components of each level) that make up the most important contextual factors as they relates specifically to SCDTDP. This framework will be referred to throughout the results section to discuss how contextual factors influenced the success of various grantees or areas of activities within the SCDTDP.

## RESULTS AND IMPACT

Grantees worked to improve care for individuals with sickle cell disease in five core areas:

1. Acute care
2. Medical home/care coordination
3. Screening and follow up
4. Transition of care
5. Hydroxyurea

The sections below are organized around these areas, each beginning with an overview of why improvement in that area is vitally important. Each section also includes descriptions of the highest-leverage changes (based on expert opinion and improvement in process measures) tested by the grantees as well as specific examples of changes that were tested. Tables are included to represent the breadth of the work completed. These tables include contextual factors that grantees found to facilitate or hinder progress.

For each of the five core areas, a set of metrics was developed to monitor progress and assess improvement. These metrics form the “measurement bank” – a set of measures that are relevant to the work of the program – and represent key care processes and outcomes in the care for individuals with sickle cell disease (For the full measurement bank, see Appendix 5). Each grantee collected data on the metrics that were most appropriate to its work. Most grantees collected data on two key outcome metrics: emergency department utilization and hospitalizations. Data from these metrics are included at the end of this section to illustrate the impact of the grantees’ improvement work. Data are represented using run charts or Shewhart charts, which show improvement over time and can identify non-random variation.

### Acute Care

Excruciating pain crises are a common experience for those with sickle cell disease, and treatment for these pain crises in the emergency department is all too often slow, ineffective, and insensitive. Timely, appropriate and respectful pain management in the emergency department can relieve pain, reduce hospitalizations, and reduce the development of chronic pain symptoms. Substantial progress was made toward decreasing the time that patients with sickle cell disease must wait to have their pain assessed (69 percent improvement) and decreasing the time between triage and the receipt of a first dose of pain medication (29 percent decrease).

Individuals with sickle cell disease can experience frequent episodes of extreme pain known as acute vaso-occlusive episodes, or pain crises. These pain crises are the most common reason for emergency department visits and hospitalizations for patients with sickle cell disease.<sup>28, 29, 30, 31</sup> Timely and appropriate use of pain medication, specifically parenteral analgesia, can relieve pain, reduce hospitalizations and reduce the development of chronic pain syndromes.<sup>32</sup> Detailed guidelines<sup>33, 34</sup> and quality indicators for the management of pain crises currently exist, but both pediatric and adult patients with the disease experience prolonged periods of waiting for pain medications in the emergency department<sup>36, 37</sup> leading to unnecessary pain, hospitalizations, chronic pain syndromes, other complications and increased health care costs. Emergency department visits and hospitalizations account for a large amount of health care expenditures in this population.<sup>38</sup>

*“When I have had to go to the emergency department, my experiences were generally not positive. Most of the time doctors and nurses didn’t trust that I was as sick as I said I was. They would not give me adequate doses of pain medications. When the doctors and nurses don’t trust me, and won’t give me adequate pain medications, the pain crisis becomes more severe. The longer and more severe the pain crisis, the longer recovery takes. So if I can go into the hospital, receive good pain control, I would need treatment for 1 day. Instead, when pain control is inadequate, my crisis worsens, and my hospital stay is increased. This means I miss more school or work.”*

**-Patient with sickle cell disease\***

\*Todd KH, Green C, Bonham VL, Haywood C, Ivy E. Sickle Cell Disease Related Pain: Crisis Conflict. The Journal of Pain, 7(7), 453-458.

At the beginning of the project, grantees and the patients and families with whom they partnered made it clear that improving acute care was a high priority due to how poorly care was managed in many emergency departments. Existing literature also indicated that acute care could be very poor.<sup>39,40</sup> As a result, acute care became one of the areas in which a large number of grantees focused their efforts.

Nine of the 15 SCDTDP and SCDNBSP grantees used quality improvement methods to improve key processes in the management of pain crises in the emergency department for adults and children. Many grantees working in the acute care setting formed multidisciplinary groups representing adult and pediatric providers drawn from the emergency department and hematology settings and included physicians, nurses, nurse practitioners, community health workers, psychologists and pharmacists. Each institution selected ideas for testing that were best suited to its own system's environments and local resources but all sites identified a physician and/or nurse "champion" who worked in the emergency department, had individual consumers review the data and provide ideas to inform the initiative, openly shared data with consumers and emergency department staff, and conducted educational efforts with both the nursing and physician staff.

Grantees worked on several changes to improve care in acute care settings. Some of the high-leverage changes that grantees tested and implemented are:

**Standard order sets:** Grantees developed, tested and implemented protocols for triage, management and medication administration. These protocols help expedite patient care and decrease delays in critical interventions such as administration of pain medication.

**Intranasal fentanyl:** Grantees tested innovative methods of pain medication delivery such as using intranasal administration (a squirt in the nose) of fentanyl which allows for rapid administration of the first dose of pain medication and ultimately more rapid pain relief. While not parenterally administered, intranasal fentanyl does result in fast and effective pain relief.

**Patient-controlled analgesia:** Patient-controlled analgesia pumps allow patients to control the timing of intravenous administration of their own pain medication, resulting in faster alleviation of pain.

### Aggregate Results in Acute Care

Across all of the grantees working in this area, substantial progress was made toward decreasing the time that patients with sickle cell disease must wait to have their pain assessed and receive a first dose of pain medication. One process improvement that helps to speed up the administration of pain medication is ensuring that all patients are given an initial pain assessment within 30 minutes of arriving in the emergency department. Grantees made significant progress in this area, achieving a 69 percent increase in the percentage of patients with sickle cell disease who had an initial pain assessment within 30 minutes, from 52 percent to 88 percent from October 2012 to May 2014 (Figure 1). Grantees saw a decrease of 29 percent in the average time from triage to first administration of pain medication, from 89 minutes at baseline to 63 minutes in May 2014 (Figure 2).

*"I am a sickle cell patient who came through the emergency room for care on Saturday evening around 6pm. I just wanted to commend your nurses and doctors who treated me that evening. [...] The doctor told me what pain medicine he was going to give me. He later asked if I felt better. I did not, so he asked what I normally get in the ER. I replied I normally get 6mg of Dilaudid but sometimes that's too much. So I suggested 3mg now and another 3 later. He said what about 4mg I said okay. I loved that he asked my opinion and continued to check with me to see if I was feeling better or not. I felt like an active part of the team. It felt very good to be treated that way in the ER. I felt calm and taken care of."*

– OH SCDTDP patient

**FIGURE 1:**

*Percentage of sickle cell disease patients who received an initial pain assessment within 30 minutes of contact (ED2)*

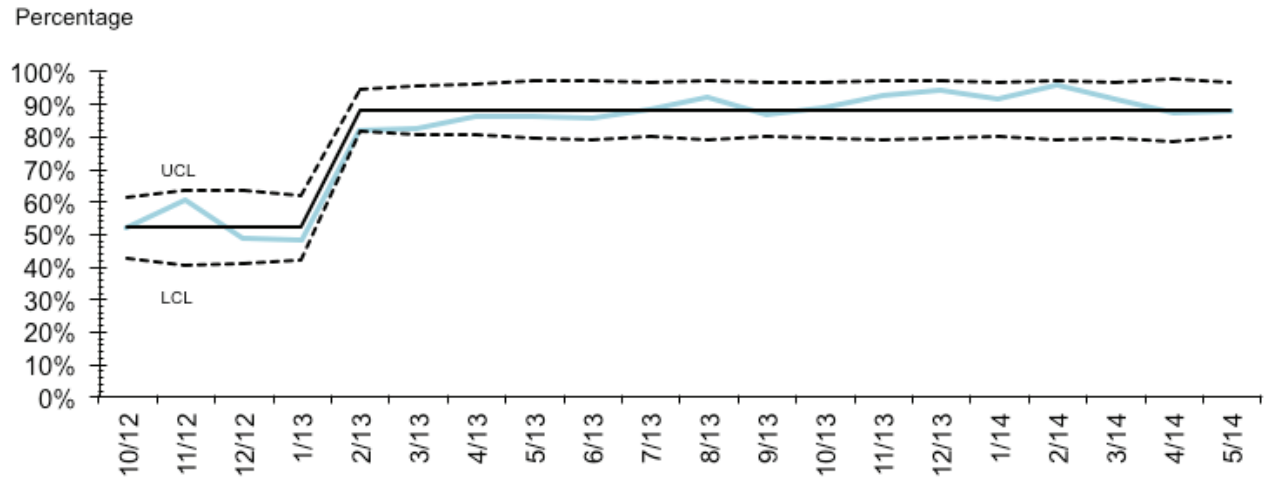


Figure 1. Shewhart chart (P Chart) representing data from CA SCDTDP, NJ SCDTDP, OH SCDTDP, PA SCDNBSP, MA SCDNBSP, OH SCDNBSP, TN SCDTDP and TN SCDNBSP grantee networks. Non-random variation exists with a shift in the mean of the data from 52 percent to 88 percent. It is also notable that these gains have been sustained for several consecutive months, suggesting that grantees have made sustainable change to their emergency departments.

**FIGURE 2:**

*Average time from triage to administration of first pain medication (ED4)*

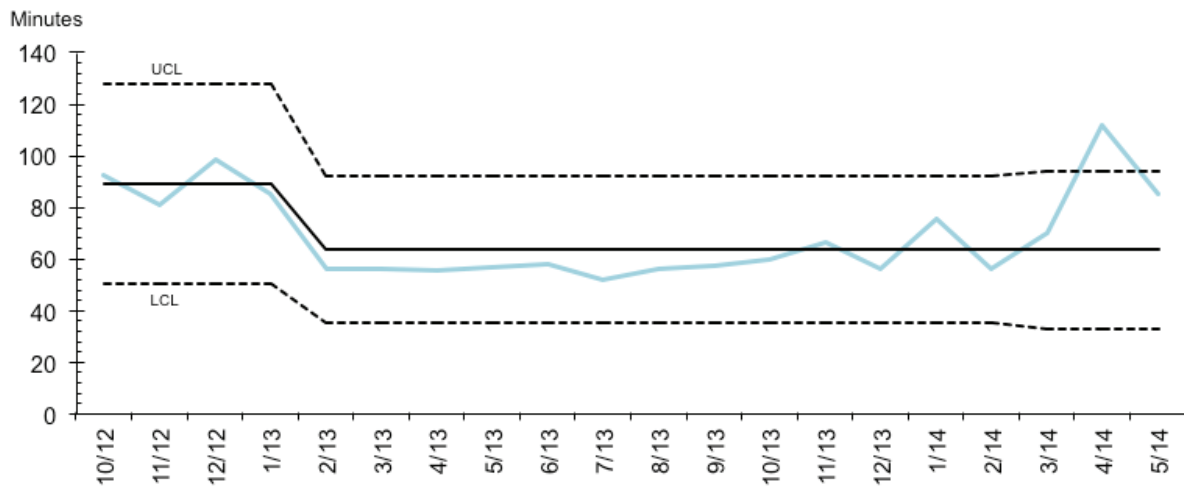


Figure 2. Shewhart chart (X-bar chart) representing data from CA SCDTDP, NJ SCDTDP, OH SCDTDP, PA SCDNBSP, MA SCDNBSP, OH SCDNBSP, TN SCDTDP and TN SCDNBSP grantee networks. These grantees saw a decrease of 29 percent in the average time from triage to first administration of pain medication, from 89 minutes at baseline to 63 minutes in May 2014. The April 2014 data point represents extreme data from one network site, which is looking into its data.

### Highlights of Grantee Work in Acute Care

The California SCDTDP grantee was a leader in improving acute care management, achieving a 61 percent decrease in the time patients wait until their first dose of pain medication. They developed a standard order set for pain management in the pediatric emergency department, which guides staff members through a procedure for treating acute pain crises and ensures that each patient receives the same pain management protocol. This standard order set led to a significant decrease in time to first administration of pain medication, from 90 minutes to 35 minutes (see Figure 3), as well as decreased time to initial pain reassessment. The California grantee network also worked to develop individualized pain action plans for their patients, which record and make easily available the specific needs of each patient. Finally, this grantee encouraged patients and families to provide feedback on their satisfaction with pain management after each visit to the emergency department so that their perspective could be incorporated into the improvement work.

**FIGURE 3**

*California SCDTDP: Average time from triage to administration of first pain medication for sickle cell disease patients (ED4)*

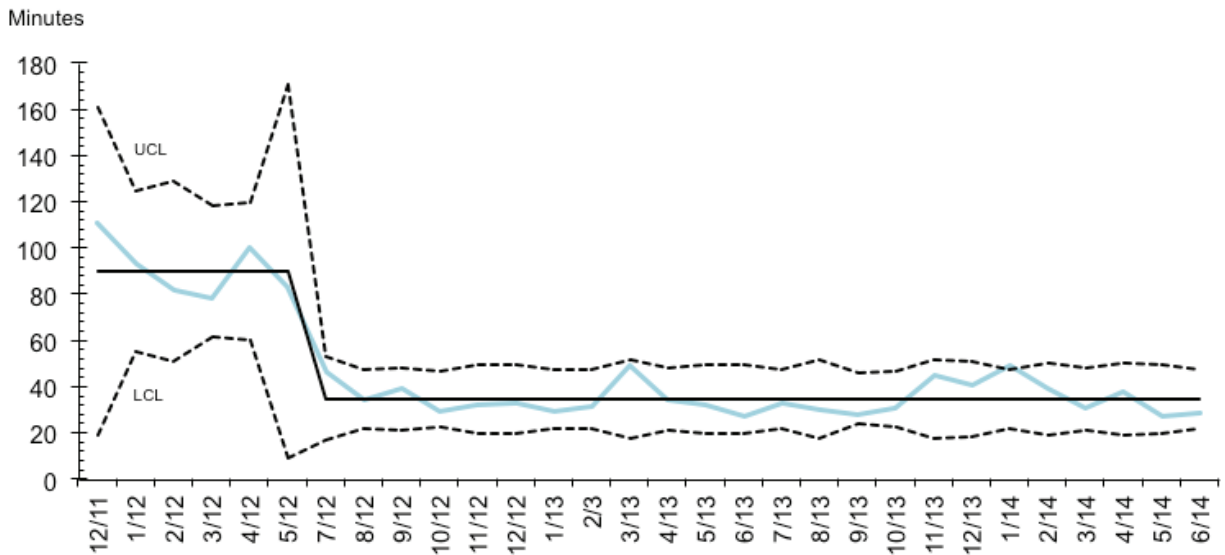


Figure 3. Shewhart chart (X-bar) representing data from the CA SCDTDP team. The team was able to achieve a 61 percent decrease in the time patients wait until their first dose of pain medication.

**FIGURE 4:**

*Massachusetts SCDNBSP: Average time from triage to administration of first pain medication for sickle cell disease patients (ED4)*

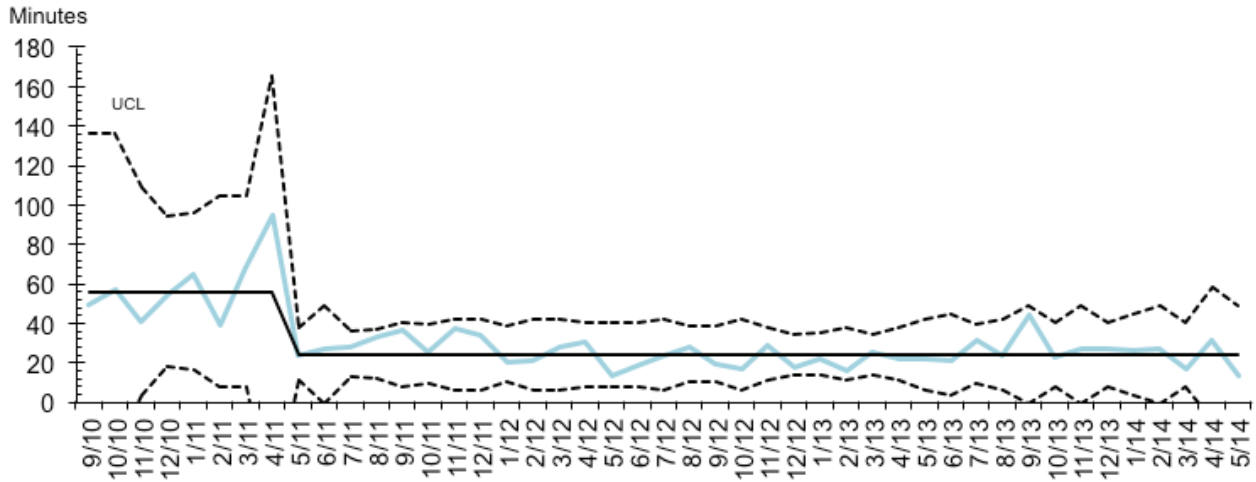


Figure 4. Shewhart chart (X-bar) representing data from the MA SCDNBSP team. This grantee was able to decrease time to first administration of pain medication from 58 minutes to 24 minutes.

The Massachusetts SCDNBSP grantee network focused on acute care in both the pediatric and adult emergency departments. They were the first grantee in the collaborative to implement the innovative intranasal administration of fentanyl for initial pain relief, which had not been widely used or studied in patients with sickle cell disease. Their other interventions include a pain medication “calculator” to assist with ensuring that patients get appropriate dosing that will alleviate their pain, early initiation of patient-controlled analgesia for admitted patients in the adult and pediatric emergency department, and use of time-directed protocols for managing pain crises in the pediatric emergency department. The grantee’s efforts led to a significant decrease in time to first administration of pain medication from 58 minutes to 24 minutes, resulting in more timely relief of patients’ pain (Figure 4). They reported high levels of buy-in from their hospital for their work in quality improvement and their efforts to improve care for individuals with sickle cell disease, in part because Boston Medical Center’s mission is to serve vulnerable populations. These contextual factors, such as high-level support and buy-in for their work, may have facilitated the success that Massachusetts SCDNBSP had in improving acute care.

The NJ SCDTDP grantee network completely revamped their emergency department check-in and triage procedures for patients with sickle cell disease. After learning that patients often waited over six hours to be triaged and treated, they reviewed procedures and found that patients were being triaged as general pain syndrome because, on arrival, patients could not select sickle cell pain as the reason for the visit. The grantee created a treatment algorithm to standardize pain management, which became standard practice and was championed by the emergency department physician leadership, and began holding yearly educational programs for all residents, physicians and nurses to reinforce the treatment protocol and the goal of timely pain relief. The grantee now meets monthly and quarterly with emergency department staff to talk about new initiatives, key patient issues (including case reviews), and individual patients whose utilization of the emergency department is high. These changes resulted in an increase in the percent of patients receiving a pain assessment within 30 minutes of triage from 20 percent to 82 percent, a 310 percent improvement over the course of the collaborative (Figure 5).



“As we know with sickle cell crises it can happen anywhere and anytime. Therefore the first place we go is the emergency room. Sadly I would have to wait anywhere between an hour to three hours. Thanks to Dr. Alice Cohen and the Central Northern NJ sickle cell network we were able to change this. They held monthly board meetings and invited the director of the emergency department and invited the patients. I am so proud and happy to have been able to voice my feelings, my experiences good and bad, and I was able to include my opinion on the things that would help the emergency room experience not to be negative or scary. I would dread going to the emergency room because I was afraid of how I would be treated by the doctors and how long I would have to wait. Now I am not afraid or apprehensive to go to the emergency room when I am in pain because my doctor and the emergency room representatives were able to come together and develop a great treatment plan for us. It is amazing to be able to walk in the emergency room and know what the protocol is because you were a part of implementing it and to know that there are people out there that care and want us to be treated fairly and not suffer in pain.”

– NJ SCDTDP patient

**FIGURE 5:**

**New Jersey SCDTDP: Percentage of sickle cell disease patients that had pain assessed within 30 minutes of triage (ED2)**

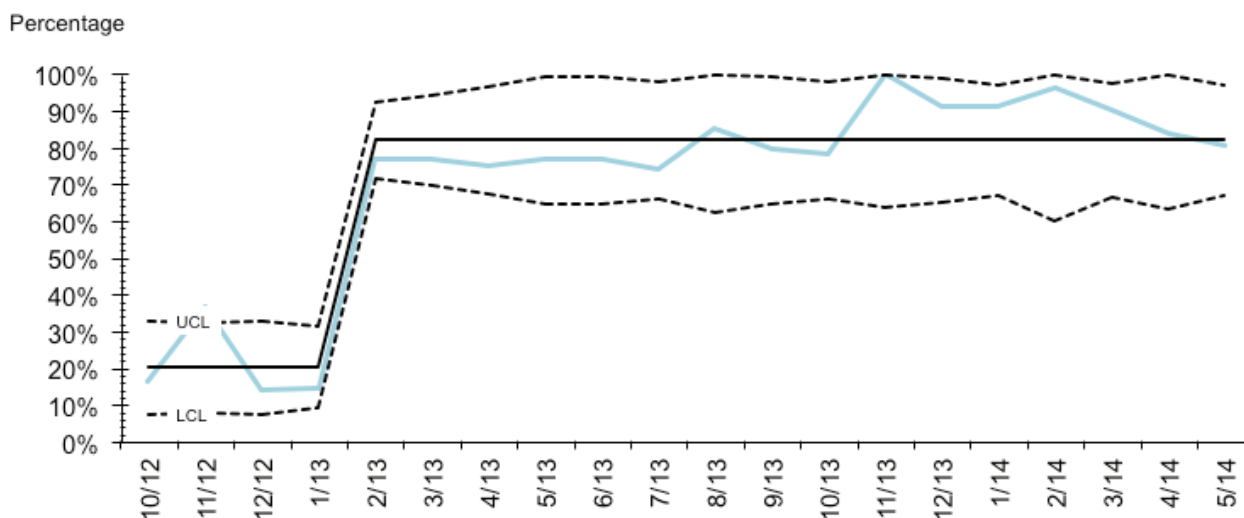


Figure 5. Shewhart chart (P Chart) representing data from NJ SCDTDP team. NJ was able to increase the percent of patients receiving a pain assessment within 30 minutes of triage from 20 percent to 82 percent, a 310 percent improvement over the course of the collaborative.

**TABLE 4:** Changes tested by grantee networks in acute care settings. \*In all tables, empty cells indicate that information was unavailable.

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
California	<ul style="list-style-type: none"> <li>• Pain medication order set in electronic health records</li> <li>• Individualized pain plans for emergency department and home use</li> <li>• Intranasal fentanyl and patient education about it, including attractive flyers and a video</li> <li>• Patient satisfaction questionnaire</li> <li>• Public data sharing</li> <li>• Ongoing emergency department and hematology staff education about the project</li> <li>• Monthly interdisciplinary quality improvement team meetings</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Committed emergency department nursing and physician champions</li> <li>• Support of emergency department and hospital quality improvement directors</li> <li>• Goals and priorities of project aligned with emergency department and hospital quality improvement initiatives</li> <li>• Emergency department protocol was driven by nursing staff</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Transition of the order set into the electronic health records created temporary difficulties. The flow of the order set was hard to follow so that times to medications and assessments increased</li> <li>• Staff changes in sickle cell clinic resulted in delay in getting pain plans finalized</li> <li>• Patient satisfaction data not consistently collected as we had to rely on students to collect the data</li> </ul>
Colorado	<ul style="list-style-type: none"> <li>• Individualized emergency department care plan</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• New observation unit/clinical pathway implemented, aligning incentives for improvement work with goals and priorities of emergency department team</li> <li>• Engaged emergency department physician champion</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Major administrative and operational changes to the emergency department</li> <li>• Engagement of emergency department champion took time</li> <li>• Large emergency department with high volume of non-sickle cell disease patients</li> </ul>
Illinois	<ul style="list-style-type: none"> <li>• Pediatric pain management algorithm</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Already had an established adult algorithm</li> <li>• Engaged pediatric emergency department physician champion</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Very limited number of emergency department visits, so little improvement data to analyze</li> </ul>
Maryland	<ul style="list-style-type: none"> <li>• Intranasal fentanyl in the pediatric emergency department</li> <li>• Public data sharing</li> <li>• New pathway for pain management in the pediatric emergency department</li> <li>• Assessment of pain management in the Sickle Cell Infusion Center for Adults</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Engaged physician champion in the pediatric emergency department</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Unable to obtain a champion in the adult emergency department</li> </ul>
Missouri	<ul style="list-style-type: none"> <li>• Time-directed algorithm for pain crises</li> </ul>	
New Jersey	<ul style="list-style-type: none"> <li>• Time-directed algorithm for pain crises</li> <li>• Team meetings regularly with emergency department team</li> <li>• Monthly tracking of data</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Two engaged emergency department physician champions (including the director of the emergency department)</li> <li>• Monthly meeting with the emergency department team attended by emergency department nursing leadership</li> <li>• Yearly education BBQ with sensitivity training in the summer for the new emergency department residents; event includes patients talking about living with sickle cell disease and the emergency department experience.</li> <li>• Resident training</li> <li>• Video training for emergency department physicians, residents and nurses</li> </ul>

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
Ohio	<ul style="list-style-type: none"> <li>Public data sharing</li> <li>Audit and feedback to emergency department nurses about appropriate visit acuity designation and care timeliness</li> <li>Monthly interdisciplinary meeting</li> <li>Annual sickle cell disease education to emergency department staff (x2)</li> <li>Celebration of emergency department team's improvements</li> <li>Implemented "Best Practice Advisory" pain reassessment alert in electronic health records</li> <li>Teamwork between emergency department staff and ambulatory staff</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Patient centered, data driven nurse leadership</li> <li>Engaged physician champion</li> <li>Staff held accountable to care timeliness expectations</li> <li>Hospital administration's focus on high emergency department utilizers</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Challenges around the emergency department going from paper to electronic health records caused project delays</li> <li>Changes in information technology delayed metric provision as routine data pulls were low priority</li> <li>High frequency of visits by small proportion of the population impacted perception of all sickle cell disease patients</li> </ul>
Pennsylvania (SCDTDP & SCDNBSP)	<ul style="list-style-type: none"> <li>Individualized pain plans</li> <li>Intranasal fentanyl as first medication for pain crises</li> <li>Pain medication "calculator" with patient-controlled analgesia for admitted patients</li> <li>Patient satisfaction questionnaire</li> </ul>	
Tennessee	<ul style="list-style-type: none"> <li>Time-directed algorithm for pain crises</li> <li>Standardized doses of pain medications implemented</li> <li>Reassessment time changed to 30 minutes after administration of IV pain medication</li> <li>Post-intervention, reassessment time reduced by 14 minutes</li> <li>Nursing staff adopted the revised protocol as an ongoing quality improvement measure</li> <li>Intranasal fentanyl research intervention underway</li> </ul>	
<b>SCDNBSP Grantees</b>		
Massachusetts	<ul style="list-style-type: none"> <li>Intranasal fentanyl as first medication for moderate/severe pain episodes</li> <li>Time-directed algorithm for pain episodes, including patient-controlled analgesia for admitted patients</li> <li>Pain medication "calculator", including recommendations for patient-controlled analgesia and oral routes</li> <li>Public data sharing</li> <li>Adult emergency department providing patient-controlled analgesia for those with care plans requiring admission</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Pediatric multidisciplinary team (emergency department physicians, emergency department nursing, pharmacy, social work, hematology, and parent) enabled the development and revision of protocols in a timely way</li> <li>Adult emergency department champion had clout to institute patient-controlled analgesia protocol</li> <li>Regular feedback sessions with physician and nursing staff led to improvements in the protocols and increased buy-in</li> <li>Dedicated grant-funded staff to do data extraction permitting continued assessment of performance</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Change in electronic health record has caused delays in reporting</li> <li>Adult emergency department unable to provide time for staff training about sickle cell disease and pain due to competing priorities</li> </ul>
Ohio	<ul style="list-style-type: none"> <li>Individualized home pain plans</li> <li>72-hour phone follow up for patients discharged from the emergency department</li> <li>Public data sharing</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Physician champion</li> <li>Quality improvement consultant and data manager to assist with documentation, tracking, and PDSAs</li> <li>Patient newsletter</li> <li>Quality improvement team who meets regularly</li> <li>Electronic health record documentation</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Initially, fellows were not aware of home pain plans</li> <li>Patient education (need for ongoing education about plans)</li> </ul>
Tennessee	<ul style="list-style-type: none"> <li>Emergency Severity Index scoring</li> </ul>	

## Medical Home/Care Coordination

Care for individuals with sickle cell disease is often fragmented and uncoordinated, leading to missed appointments, poor medication adherence, and inconsistent provision of recommended components of care such as screenings and immunizations. Well-coordinated care in the context of a medical home, including support for chronic illness self-management, can lead to fewer and less severe complications of sickle cell disease. The coordination of primary and specialty care was improved in many areas, including the percentage of patients who were evaluated by a hematologist within the past year (increase of 135 percent) and the percentage of patients whose care plans were reviewed during their visit (increase of 170 percent).

Care for persons with sickle cell disease is often fragmented, spanning multiple providers and often multiple institutions. This results in many persons with sickle cell disease not having a medical home that coordinates their care. A patient-centered medical home is an approach to providing comprehensive primary care for children, adolescents and adult that is patient- and family-centered, comprehensive, coordinated, accessible and committed to quality and safety.<sup>41</sup> The location of the medical home for individuals with sickle cell disease may vary based on patient and family preferences, and proximity to primary care and specialty care providers.<sup>17,42</sup> One study highlighted that many children with sickle cell disease did not have care that met the standards for a patient-centered medical home.<sup>13</sup> Additional literature has also shown that patients who receive comprehensive care had fewer emergency department visits and hospitalizations.<sup>43</sup> Coordination between primary and specialty care is crucial to the provision of high quality care for patients with sickle cell disease, as the lack of regular ambulatory care may lead to increased health care utilization in acute care settings (including increased reliance on the emergency department, particularly among transition age youth (ages 12-25) and adults<sup>44</sup>) as well as missed opportunities for preventive care. Lack of outpatient hematology follow up after hospital discharge is a known risk factor for 30 day readmission among individuals with sickle cell disease.<sup>45</sup>

One particularly important area of care coordination is the promotion of chronic illness self-management, which is crucial to improving outcomes of children and adults with sickle cell disease.<sup>46</sup> Patients and families have a central role in managing their own or their child's health, and engaging in healthy behaviors such as adhering to prescribed medication, eating healthy foods, drinking plenty of fluids, staying active, avoiding extreme temperatures, and managing stress levels. These behaviors can lead to fewer and less severe complications such as pain crises, and thus improve outcomes and improve quality of life. Knowing how to manage mild complications at home and when to appropriately seek health care also contributes to improved quality of life and may lead to lower health care utilization costs. Comprehensive care and pain management plans, important tools for self-management focused on increasing individuals' self-efficacy in managing their disease, also facilitate care coordination by ensuring that patients and providers operate from a common written document. Productive interactions between informed and empowered patients and a prepared and proactive practice team led to improved patient outcomes.<sup>47</sup>

Grantees made improvements across multiple dimensions of care, from the coordination of care across multiple health systems and networks to the provision of key elements of primary and specialty care including immunizations, transcranial Doppler screenings, annual dilated eye exams, and administration of prophylactic penicillin. All 15 SCDT-DP and SCDNBSP grantees made improvements in the realm of care coordination, and many leveraged pre-existing relationships in their networks and developed new relationships over the course of the collaborative to expand and extend both clinical and psychosocial services. Grantees were able to improve processes to increase the speed and ease with which patients were able to access health services, as well as address some of the psychosocial issues that are often seen in this population, including mental health issues, unemployment, and homelessness. Many grantees also implemented changes that aimed to increase the support that patients receive for self-management.

The high-leverage changes that grantees tested were:

- **Increase access to primary care providers:** Primary care providers can help to coordinate care for patients with sickle cell disease and provide care for general health maintenance and concerns. Eight grantees worked to ensure that patients have primary care providers and – importantly – attend appointments with their primary care provider using various approaches such as phone call and text reminders, dedicated appointment times for studies and tests, and accompanying young adults on their first visit(s) to the adult sickle cell program.

- **Provision of recommended care:** Grantees worked on coordinating, assessing, and tracking the recommended components of care for patients with sickle cell disease (e.g., transcranial Doppler screenings, immunizations, screening for and treatment of mental health needs, etc.) using electronic health records, checklists and pre-clinic multidisciplinary team meetings.
- **Individualized care plans:** Several grantees developed templates for individualized care plans and pain treatment plans, some of which were included in their respective electronic health records. Individualized pain management plans in the emergency department are effective in delivering high quality management of pain both at home and in the emergency department and are associated with a high level of patient satisfaction and decreased avoidable hospitalizations.<sup>48</sup>
- **Patient navigators or community health workers:** Grantees also engaged patient navigators or community health workers to help patients to coordinate all aspects of clinical care and address psychosocial needs including insurance, housing, and employment.
- **Patient training:** Several grantees used Stanford University's Chronic Disease Self-Management Program, which builds confidence, empowerment and decision-making skills in addition to training on specific topics.
- **Provider education:** Education programs such as the ACCEPT program (Advancing Communication and Care by Engaging Patients in Training), were used to train providers to integrate self-management support strategies (such as goal-setting) into routine clinical care.
- **Tracking tools:** Tracking information through tools such as patient event diaries and electronic health record templates make it easier to document information and ensure communication between patient and providers.

### *Aggregate Results in Medical Home/Care Coordination*

Overall, the collaborative improved the coordination of primary and specialty care across multiple dimensions. Grantees were able to improve the percent of patients who had visits with their hematologists and primary care providers and the percent of patients who had their care plans reviewed. The percent of patients who had an evaluation with a hematologist increased from a mean of 40 percent from January 2012 to May 2013 to a mean of 94 percent from June 2013 to June 2014, an improvement of 135 percent throughout the collaborative (Figure 6). The percent of patients whose care plans were reviewed improved from a mean of 33 percent to a mean of 89 percent, an improvement of 170 percent throughout the collaborative (Figure 7). Improvement is also seen in the percent of patients who had a documented visit with their primary care provider (Figure 8).

**FIGURE 6:**

*Percent of patients with evaluation by a hematologist within the past 12 months (Medhome 1)*

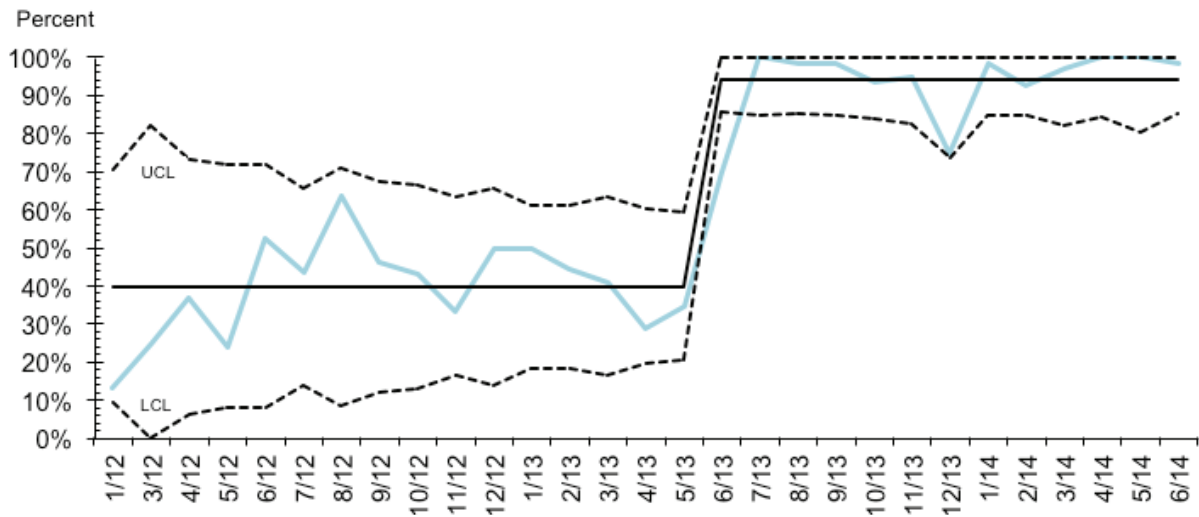


Figure 6. Shewhart chart (P chart) representing data from NJ TDP, NY NBSP, OH NBSP, and OH TDP grantee networks. Improvement can be seen from a mean of 40 percent to a mean of 94 percent, representing an improvement of 135 percent throughout the collaborative.

**FIGURE 7:**

*Percentage of patients whose care plan was reviewed during visit (Medhome7)*

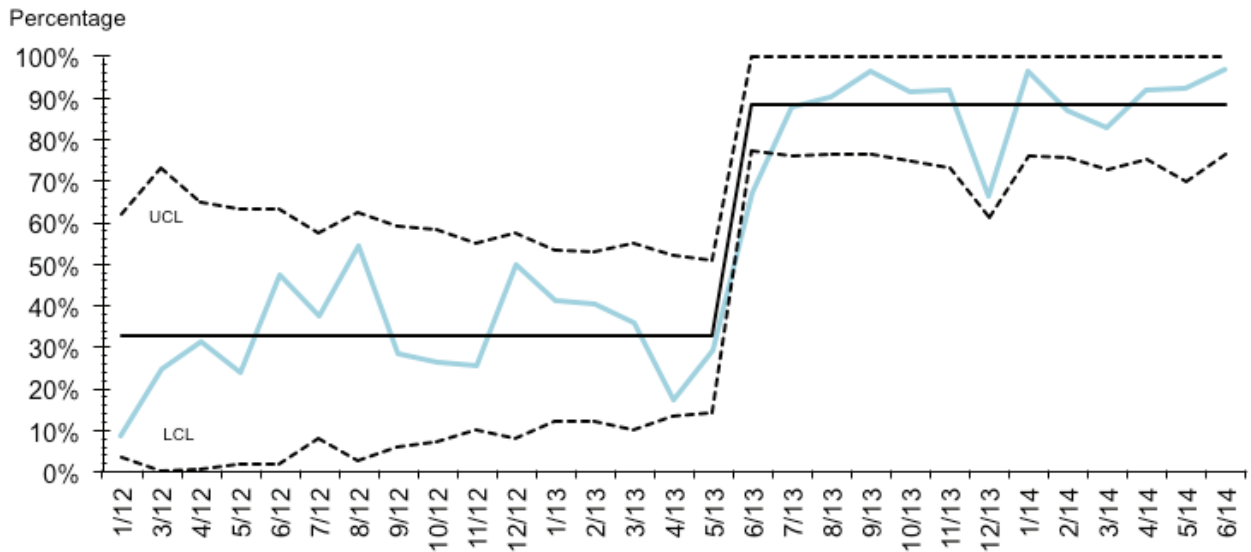


Figure 7. Shewhart chart (P chart) representing data from NJ SCDTDP, NY SCDNBSP, OH SCDNBSP, and OH SCDTDP grantee networks. Improvement can be seen from a mean of 33 percent to a mean of 89 percent, representing an improvement of 170 percent throughout the collaborative.

**FIGURE 8:**

*Percentage of patients with a documented primary care provider visit (Medhome 4)*

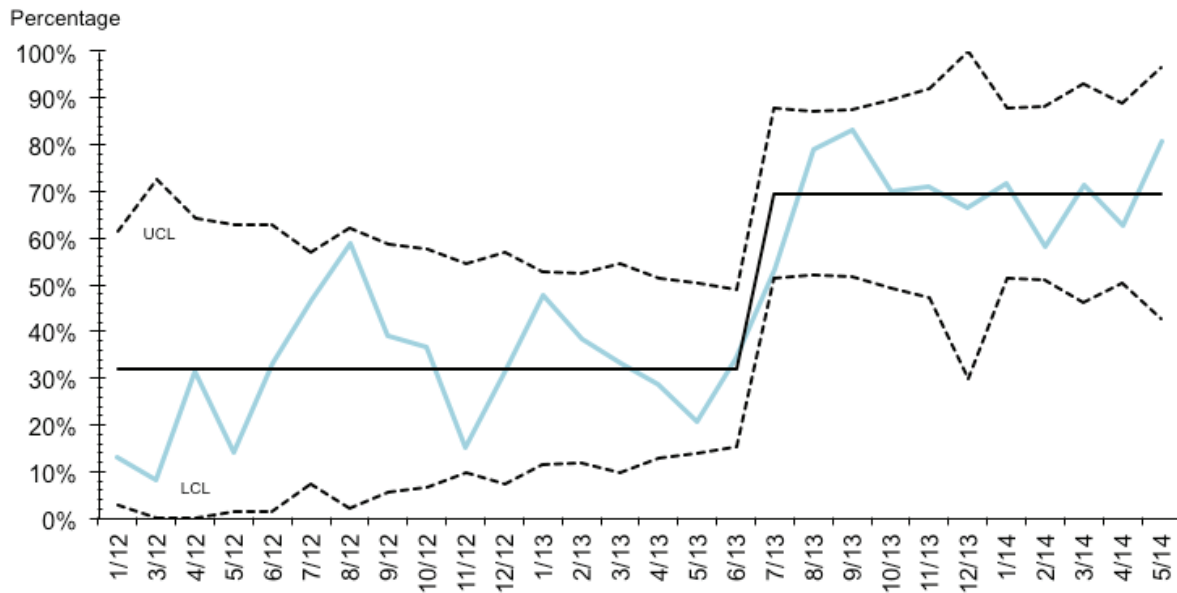


Figure 8. Shewhart chart (P chart) representing data from NJ SCDTDP, NY SCDNBSP, OH SCDNBSP, and OH SCDTDP grantee networks. Improvement can be seen from a mean of 33 percent to a mean of 70 percent, representing an improvement of 119 percent throughout the collaborative.

*Highlights of Grantee Work in Medical Home/Care Coordination*

The California SCDTDP grantee network improved self-management by working with and training individuals with sickle cell disease and parents as peer health coaches to provide self-management support (e.g., addressing adherence with oral iron chelation). This grantee also tested the use of a new technology, Glow caps® on medicine bottles that light up as a reminder to take the medicine with-in, to increase medication adherence. The health coaches also worked with patients to overcome barriers to accessing care (e.g., assist with filling out insurance forms, finding transportation, child care, housing, etc.). In the newest phase of the project, the sickle cell social worker was trained in motivational interviewing, an evidence-based practice that focuses on exploring and resolving ambivalence and centers on motivational processes within the individual that facilitate change. This network also worked to increase the number of pediatric patients ages 8-21 years who have a “pain action plan” for continuity of management between home, school, the clinic, the emergency department, and inpatient care (Figure 9). The plans are developed through the input of a patient’s family, clinical team, and psychologist, and includes both pharmacological and non-pharmacological strategies. Families receive a printed copy of the plan that focuses on how to stay healthy, as well as how to deal with mild, moderate and severe pain.

*“My health coach helped me out the most. The fact I received reminders helped me... Without that I would have continued taking Exjade [medication to help manage iron overload] only 3 days per week. Group meetings with other patients and coaches, helped [me] to recognize it wasn’t only me who faced [these] difficulties.”*

– CA SCDTDP patient

**FIGURE 9:**

*California SCDTDP: Percentage of patients with finalized pain plans*

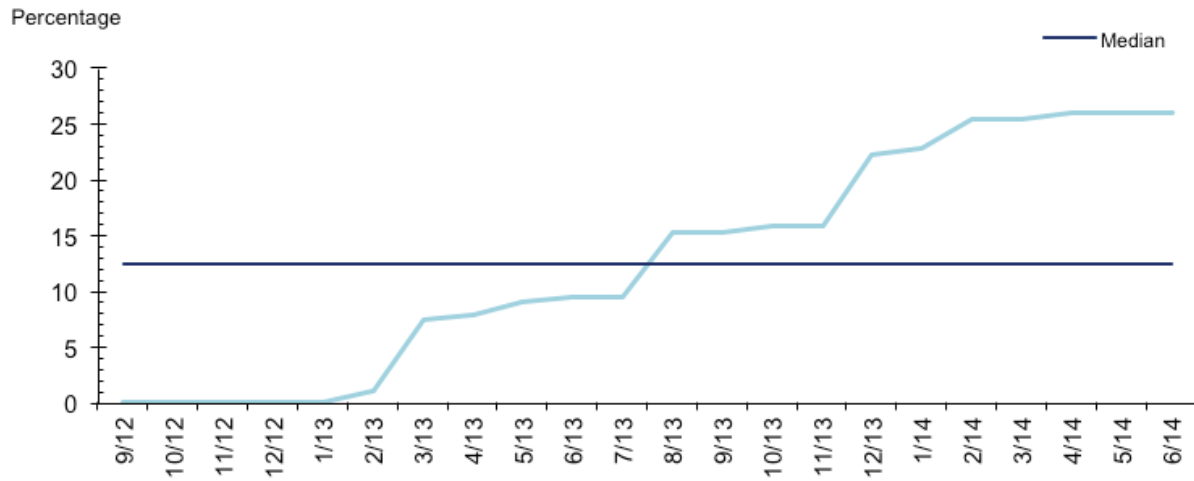


Figure 9. Run chart representing data from CA SCDTDP team. CA was able to improve the percent of patients with finalized pain plans from 0 percent at baseline to 25 percent.

The Colorado SCDTDP grantee network focused on improving care coordination using several strategies, including using six trained patient navigators (several of whom are family members and/or caregivers), integrating sickle cell disease specific templates in their electronic health record system, and conducting focus groups/key informant interviews with primary care providers about their preferences around coordinating care for individuals with sickle cell disease. This last was in order to help develop a care coordination template that could be used in the electronic health records to promote shared care. Some of the patient navigators who were provided with formal training through Colorado SCDTDP funding now participate in the Statewide Patient Navigator Working Group, developing strategies to enhance sustainability and develop quality metrics for patient navigators and navigation programs statewide.

**FIGURE 10:**

*California SCDTDP: Percentage of patients with finalized pain plans*

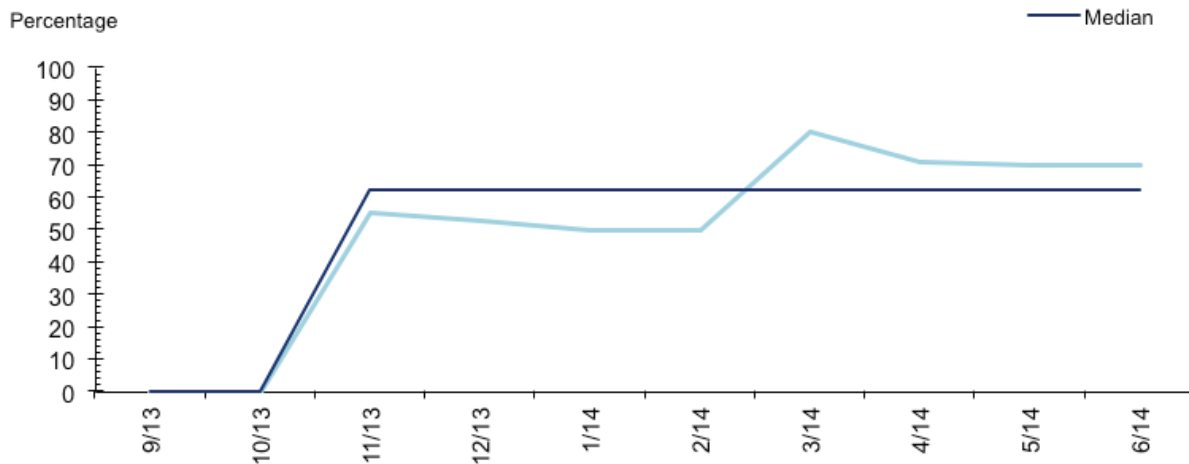


Figure 10. Run chart representing data from the CO SCDTDP team. CO was able to improve the percent of patients with a care coordination plan from 0 percent at baseline to a median of 63 percent.



The Illinois SCDTDP grantee network has addressed several dimensions of care to help streamline and coordinate care for individuals with sickle cell disease at their federally qualified health center, at the clinic in Peoria, and within their network. Interventions include care coordination tools and needs assessment checklists, that include both clinical (e.g., immunizations, transcranial Doppler, etc.) and psychosocial (e.g., insurance, housing, mental health screening, etc.) components of overall health integrated into a process that is client-friendly and not intimidating, resulting in a higher degree of patient comfort and involvement. Their care coordination tool is now used with 70 percent of their patients (see Figure 11) and 100 percent of their patients are receiving their recommended annual transcranial Doppler screening (see Figure 12).

**FIGURE 11:**

*Illinois SCDTDP: Percentage of patients completing care coordination tool*

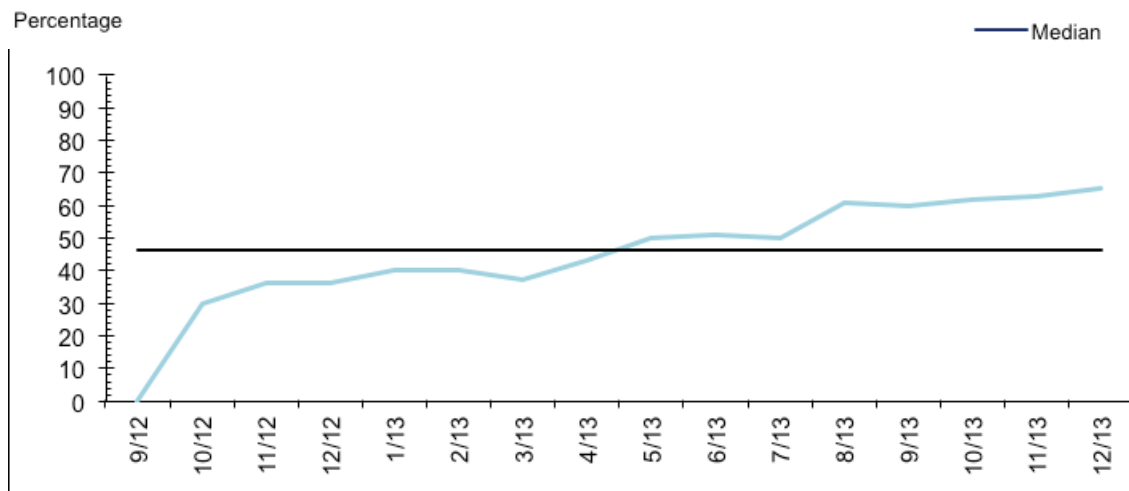


Figure 11. Run chart representing data from IL SCDTDP team. The team was able to increase the use of their care coordination tool so that it is now used with 70 percent of their patients.

**FIGURE 12:**

*Illinois SCDTDP: Percentage of patients with a transcranial Doppler screening in the past 12 months (Hemecare 3)*

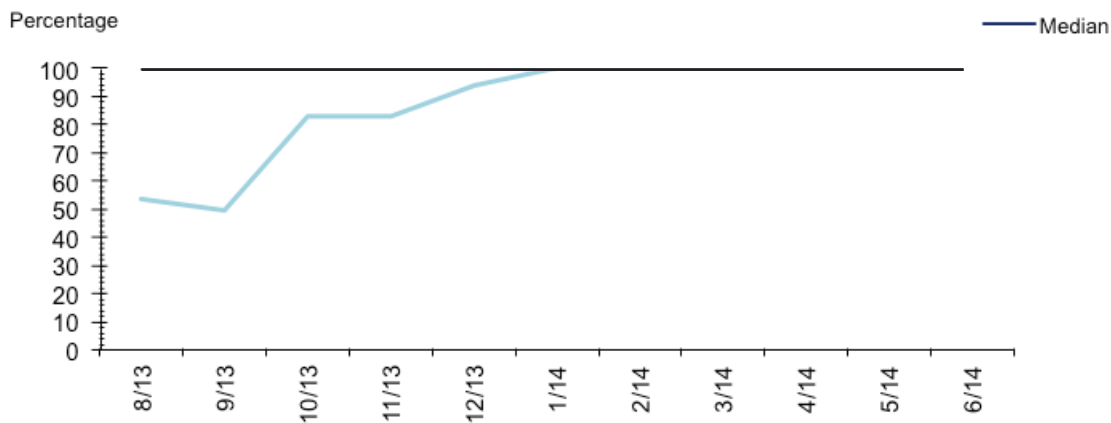


Figure 12. Run chart representing data from the IL SCDTDP team. The team was able to improve the percentage of patients who received a transcranial Doppler screening in the past 12 months from 55 percent at baseline to 100 percent, representing an improvement of 81 percent over 11 months.

The Maryland SCDTDP grantee network began to provide much-needed dental care to adult patients without dental insurance who were referred by the sickle cell program’s social worker. The network paid for visits to a private dentist for services such as cleanings, fillings, x-rays, and single extractions, and served 56 unique patients overall, with 153 appointments completed. In addition to the real benefit to patients, the grantee is investigating whether receiving dental care affects patients’ rates of emergency department visits and hospitalizations by examining patients’ medical records for 12 months before and after the initial dental appointment. The grantee has collected some data and when the analysis is complete, will be able to assess whether the dental program affected patients’ acute care visits or hospitalizations (including but not limited to those for uncomplicated sickle cell pain).

The New York SCDNBSP grantee network developed a patient event diary that allows patients or families to record all events of medical significance, including pain, fever and other symptoms, which enables important health information to be shared easily with the patient’s team of providers. The event diary also provides essential information on ways to stay healthy, how to react to various complications of sickle cell disease and specific information about the patient’s care plan.

The Ohio SCDNBSP grantee network worked to increase the number of sickle cell patients ages 5-21 who have a home pain management plan. The plans are developed through the input of a patient’s family, clinical team, and psychologist, and include both pharmacological and non-pharmacological strategies to address mild and moderate pain at home, allowing patients to avoid unnecessary visits to the emergency department. Families receive a printed copy of the plan, it is documented in the electronic health record, and it is updated as needed.

The Ohio SCDTDP grantee network worked to develop a consistent process to develop and document collaborative treatment plans. The network coordinated care through chart review and pre-clinic, multi-disciplinary meetings, and used a template in their electronic health record that was tailored to sickle cell disease (a collaborative treatment plan template) that was reviewed and printed for each patient at the time of the visit. They also used a community health worker to improve patient self-management, appointment completion, and adherence to treatment plans. In addition, the network trained nearly 20 members in a self-management support protocol, ACCEPT, which is focused on motivational interviewing and goal setting.

**TABLE 5:** Changes tested by grantee networks in medical home/care coordination

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
California	<ul style="list-style-type: none"> <li>• Mental health screening</li> <li>• Community health worker/patient navigator</li> <li>• Care coordination tool - pain plans for home, emergency department and inpatient</li> <li>• Improved access to care by offering transportation assistance and childcare</li> </ul>	<p><i>Facilitators</i></p> <ul style="list-style-type: none"> <li>• Perceived value of mental health screening high</li> <li>• Patient navigator highly skilled (retired Master’s level) individual with sickle cell disease</li> <li>• Youth with sickle cell disease and their parents surveyed individually or in focus groups and rated pain action plans as highly attractive</li> <li>• Core pediatric and adult sickle cell specialists very knowledgeable and committed</li> </ul> <p><i>Barriers</i></p> <ul style="list-style-type: none"> <li>• Spread of mental health screening is limited by the lack of follow-up mental health resources in the community</li> <li>• Challenges with state insurance (i.e., cumbersome application process) took the focus of the patient navigator away from other needs as she focused exclusively for a time just on getting the applications submitted</li> <li>• It took time to bring new pediatric sickle cell center staff up to speed</li> </ul>

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
Colorado	<ul style="list-style-type: none"> <li>• Primary care provider referrals/appointments</li> <li>• Patient navigator</li> <li>• Training primary care providers, patient/caregiver partners in care of individuals with sickle cell disease</li> <li>• Sickle cell disease care summary plan for communication between specialist and primary care provider</li> <li>• Fever educational materials for individuals with low health literacy</li> <li>• Electronic health record templates</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Interest in patient navigation</li> <li>• Availability of patient navigation training materials and a training program</li> <li>• New electronic health record system implemented on the adult side, allowing for creation of “smart text” and templates in notes</li> <li>• Specialty care providers very committed and motivated to outreach to primary care providers</li> <li>• Interest in sickle cell disease by primary care providers</li> <li>• Specialty care very centralized at tertiary academic medical center; pediatric and adult specialists and care teams very knowledgeable and connected to statewide activities and sickle cell disease population</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Lack of communication and coordination across settings due to HIPAA and different EHRs for pediatric and adult hospitals</li> <li>• Challenging to find providers willing to accept Medicaid due to low reimbursements</li> <li>• Differential acceptance of health insurance programs across care providers and settings</li> <li>• Lack of mental health support and dental care</li> <li>• Low sickle cell disease patient density</li> </ul>
Illinois	<ul style="list-style-type: none"> <li>• Primary care provider referrals/appointments</li> <li>• Care coordination tool and needs assessment checklist</li> <li>• Tracking and coordination of transcranial Doppler screening</li> <li>• Tracking and coordination of immunizations in pediatric patients</li> <li>• Pre-clinic care coordination using sickle cell disease-specific patient visit template</li> <li>• Mental health screening</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• New tracking tool for transcranial Doppler screening, which made it easy to map the failed appointments and get them re-scheduled promptly</li> <li>• Tracking of immune status as one review topic at every pre-clinic meeting</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Limited number of adult practices that accept patients and have knowledge of sickle cell disease</li> <li>• Complexity of having to obtain past records of immunizations from primary care offices (sometimes multiple) and public health departments</li> </ul>
Maryland	<ul style="list-style-type: none"> <li>• Primary care provider referrals/appointments</li> <li>• Specialty care referrals/appointments (dental)</li> <li>• Community health worker coordinates care</li> <li>• Training primary care providers in care of individuals with sickle cell disease</li> <li>• Referral to social support</li> <li>• Developed videos to explain the importance of a primary care provider for patients with sickle cell disease</li> <li>• Provided transportation to primary care provider appointments</li> </ul>	<p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Challenging to engage primary care providers in training when they had small number of sickle cell disease patients in their panel</li> <li>• Difficulty with engaging federally qualified health centers</li> </ul>
Missouri	<ul style="list-style-type: none"> <li>• Primary care provider referrals/appointments</li> </ul>	
New Jersey	<ul style="list-style-type: none"> <li>• Health maintenance form to track screenings, eye exams, and vaccinations</li> <li>• Mental health screening tool and hospital-wide policy</li> <li>• Community health worker coordinates care and makes reminder calls</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Provided bus tickets for patients to attend clinic to facilitate access to care</li> <li>• Social worker and community health worker go with patients to Medicaid/Social security office to assist with completion of applications for health insurance</li> <li>• Social worker created a community resource guide and insurance information guide for patients</li> </ul>

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
Ohio	<ul style="list-style-type: none"> <li>• Interdisciplinary team: primary care provider, hematologist, and pharmacy providers, nurses, APNs, social worker, psychology student, and community health worker involved regularly</li> <li>• Weekly care coordination meetings and associated chart review/needs assessment</li> <li>• Standardized electronic health record tool (sickle cell disease-specific EPIC collaborative treatment plan template "SMART Phrase")</li> <li>• Patient collaboration in treatment plan at time of visit</li> <li>• Primary care provider referrals/appointments</li> <li>• Care coordinator</li> <li>• Referrals for recommended preventative services</li> <li>• Partnerships with ophthalmology and dental services in academic sites for preferred patient access</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Supportive hematology and primary care medical directors</li> <li>• Existing structure for interdisciplinary teams at academic medical center</li> <li>• Local grant support for community health worker</li> <li>• Community health worker's background (10+ years) at academic medical center</li> <li>• Engagement of patients as advocates in healthcare provider education sessions</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Data collection for outcomes is labor intensive as there is no automated reporting due to electronic health record transition</li> <li>• Use of multiple and non-interfacing electronic health record systems</li> <li>• Lack of understanding of community health worker role</li> </ul>
Pennsylvania (SCDTDP & SCDNBSP)	<ul style="list-style-type: none"> <li>• Primary care provider referrals/appointments</li> <li>• Community based care coordinator</li> <li>• Referral to social support</li> </ul>	
Tennessee	<ul style="list-style-type: none"> <li>• Implementing and advertising a Medical home</li> <li>• Vision screenings</li> <li>• Monthly interdisciplinary care coordinator meetings</li> <li>• School meetings IEP/504 plans</li> </ul>	
<b>SCDNBSP Grantees</b>		
Illinois	<ul style="list-style-type: none"> <li>• Pediatric pain management algorithm</li> </ul>	
Massachusetts	<ul style="list-style-type: none"> <li>• Registry and monthly reports track vaccinations, transcranial Doppler screening, and hydroxyurea use in pediatric patients</li> <li>• Track primary care provider referrals/appointments for adults with sickle cell disease, mental health screening for children with sickle cell disease</li> <li>• Track adults with sickle cell disease who had follow-up hematology appointments made within 28 days of discharge, within 14 days if possible; also track appointments kept</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Champions supported quality improvement efforts both in pediatrics and internal medicine</li> <li>• Harnessed IT systems to generate regular reporting from the registry</li> <li>• Patient navigators assisted with making primary care provider referrals/appointments, mental health screening</li> <li>• Grand rounds given for internal medicine and family medicine providers led to increased interest (and appointments) to care for adults with sickle cell disease</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Patient navigators are grant-funded, need alternative funding streams to maintain work</li> <li>• Need additional clinic slots for adult sickle cell disease patients to allow timely follow-up post-discharge</li> </ul>
New York	<ul style="list-style-type: none"> <li>• Time-directed algorithm for pain crises</li> </ul>	<p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Transcranial Doppler screening offsite</li> </ul>
Ohio	<ul style="list-style-type: none"> <li>• Track and coordinate transcranial Doppler Screening</li> <li>• Care coordinator</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Multidisciplinary team including child life, behavioral medicine and hematology</li> <li>• Engaged parents</li> <li>• Quality improvement consultant and data manager to assist with documentation</li> <li>• Care manager champion</li> <li>• Quality improvement team who meets regularly</li> <li>• Zoo Day event where we can educate parents</li> <li>• Video support &amp; Institutional YouTube channel</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Staff turnover</li> <li>• Change in quality improvement support team</li> <li>• Technology problems resulted in loss of some documentation</li> </ul>

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDNBSP Grantees</b>		
Tennessee	<ul style="list-style-type: none"> <li>• Primary care provider referrals/appointments</li> <li>• Patient management checklist</li> <li>• Mental health screening</li> </ul>	

**TABLE 6:** Changes tested by grantee networks in self-management

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
California	<ul style="list-style-type: none"> <li>• Peer health coaches</li> <li>• “Glow caps” (specialized pill bottles whose caps light up to remind patients to take their medication)</li> <li>• Pain plans for home, emergency department and inpatient</li> <li>• Stanford University’s Chronic Disease Self -Management Program</li> <li>• Motivational interviewing (a counseling approach that helps people set and achieve personal goals)</li> <li>• Created self-management support materials in hard copy and posted on website (some materials translated into Spanish)</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• The introduction of the technology of the Glow caps highlighted the need for greater staff involvement with regard to self-management support, hence the addition of motivational interviewing</li> <li>• Received another grant to continue study of motivational interviewing</li> <li>• Glow caps provided free of charge</li> <li>• Hospital communications department contributed design expertise for materials</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Volunteer peer health coaches were not able to be as available for support as patients needed</li> <li>• Competing priorities/negative social determinants of health (e.g., housing, transportation, trauma exposure) made it difficult for patient and families to fully engage in self-management</li> </ul>
Illinois	<ul style="list-style-type: none"> <li>• Life skills coaching</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Several staff and one very active patient partner got trained early on in the programs, to facilitate its spread to others</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Even though fairly extensive methods used to advertise the training, few patients came to sessions</li> </ul>
Maryland	<ul style="list-style-type: none"> <li>• Community health workers assisted in the development of self-management skills</li> <li>• Written pain management plans for adult patients</li> </ul>	
New Jersey	<ul style="list-style-type: none"> <li>• Primary care provider referrals/appointments</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Community-based organization’s coordination of a self-management program for patients across the state</li> <li>• All partnering community-based organizations have yearly education programs and our team members and physicians provide education to patients and families – this has included adult and pediatric physicians, emergency department physicians, sickle cell nurse coordinator, community health workers and psychosocial professionals</li> </ul>
Ohio	<ul style="list-style-type: none"> <li>• Motivational interviewing</li> <li>• ACCEPT provider self-management protocol</li> <li>• Coaching and follow up by community health worker</li> <li>• Stanford University’s Chronic Disease Self -Management Program</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Participation of key project staff in other endeavors that use and teach the ACCEPT provider self-management protocol</li> <li>• Local grant support for community health workers</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Negative social determinants of health(e.g., lack of housing, unsafe physical environments, no access to healthy foods) make it difficult for some patients to fully engage in self-management</li> </ul>
Pennsylvania (SCDTDP & SCDNBSP)	<ul style="list-style-type: none"> <li>• Life skills coaching</li> <li>• Pain management plan</li> </ul>	

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
Tennessee	<ul style="list-style-type: none"> <li>• Text messaging reminder for medications tailored to adolescents</li> <li>• Intensive teen clinic protocol; education and skills for teens twice a year for 7 years (12-18 years old)</li> <li>• Teens practice to complete adult health record document – completes at 15, 16 &amp; 17 years old</li> <li>• Teens completes career inventory at 14 years old</li> <li>• Teens complete academic plan for at 16 years old</li> </ul>	
<b>SCDNBSP Grantees</b>		
Illinois	<ul style="list-style-type: none"> <li>• Take Charge of Your Health program</li> <li>• Life skills coaching</li> <li>• Stanford University's Chronic Disease Self-Management Program</li> </ul>	
New York	<ul style="list-style-type: none"> <li>• Patient event diary</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Grant funding supported creation of patient event diary</li> </ul>
Ohio	<ul style="list-style-type: none"> <li>• Pain action plan</li> <li>• ACCEPT provider self-management protocol</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Quality improvement team that meets regularly</li> <li>• Design students from the University of Cincinnati</li> <li>• Institutional support and coaching for ACCEPT provider self-management protocol</li> <li>• Integration of pain action plan into electronic health record patient instructions</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Institutional change in ACCEPT provider self-management protocol</li> <li>• Change in documentation process in electronic health records</li> <li>• Institutional process for patient education materials</li> </ul>
Tennessee	<ul style="list-style-type: none"> <li>• Pain management plans</li> </ul>	

## Screening and Follow up

Early identification and proper follow-up care and counseling is important for individuals with sickle cell disease and sickle cell trait, but newborn screening systems vary greatly state to state and infants with a positive screen can be lost to follow up. Strong screening and follow-up systems have many long-term benefits, including reduced mortality of children with sickle cell disease (from the use of preventative medication) and the ability of those with sickle cell disease and trait to make informed reproductive choices (from genetic counseling). Grantee networks have worked with providers, genetic counselors, families, and state departments of public health to ensure that families receive notification of positive screens and follow up care is provided. They have also reached out to immigrant populations and provided free testing and counseling at a variety of community events.

Early studies documented that the early administration of penicillin prophylaxis reduced the incidence of pneumococcal infections by 84 percent and reduced mortality from such infections in children with sickle cell disease.<sup>20</sup> This finding provided the rationale for newborn screening and early diagnosis (in the newborn period) to ensure prompt treatment of affected individuals.<sup>20</sup> The result of screening performed in the neonatal period has immediate implications for the infant found to have the disease, but also longer-term implications for both the child and other family members, such as the ongoing need for genetic counseling and education.<sup>49</sup>

Only since May 1, 2006 have all US states and the District of Columbia required and provided universal newborn screening for sickle cell disease, which also identifies sickle cell trait, despite a national recommendation to this effect in 1987.<sup>50</sup> Each state has developed a newborn screening program that meets the needs and resources of the state. For sickle cell disease and sickle cell trait, some states have well-developed follow-up programs in which nurses, program specialists or community-based organizations contact families of infants with positive newborn screening results

and, as necessary, arrange confirmatory testing and follow up with specialists and genetic counselors.<sup>51</sup> Other states rely on the primary care provider to arrange for confirmatory testing, provide education to parents and referral to specialists.<sup>52</sup> Variation also exists in the process of screening individuals who are not screened as infants including pregnant women and immigrants.

Many grantees have partnered across their communities to incorporate screening, free testing, genetic counseling and education into their outreach work. This has allowed them to reach wide, diverse populations, especially emerging populations such as recent immigrants not screened in the newborn period. This is important so that individuals with sickle cell trait are aware of their status and can make informed reproductive decisions (since the child of two parents with sickle cell trait has a 25 percent chance of inheriting sickle cell disease), as well as be aware of rare medical conditions resulting from sickle cell trait. Grantees developed prompts in their electronic health records and other methods to alert providers that genetic counseling was needed during adolescence.

The high-leverage changes that grantees tested were:

- **Education and follow up:** Grantees also worked with their networks and with their respective state Departments of Public Health to improve follow up and education after identification of sickle cell trait and confirmation of sickle cell disease. These strategies include providing counseling and education over the phone, group clinic visits for newborns with sickle cell disease and connecting with primary care providers to ensure appropriate follow up.
- **Sickle cell trait toolkit:** The grantees involved in the Screening Affinity Group collected and shared resources to develop a sickle cell trait toolkit to help providers follow up with individuals and families after identification of sickle cell trait, so that they are prepared to offer excellent, evidence-based care to this population.

### *Highlights of Grantee Work in Screening and Follow Up*

The Illinois SCDNBSP grantee network, often working together with the Illinois SCDTDP grantee network, has focused extensively on newborn screening follow up and education, working to ensure that families receive notification from the Illinois Department of Public Health, and then share visual, auditory, and kinesthetic educational materials and conduct education and counseling over the phone. These steps are designed to make certain that the families diagnosed with sickle cell disease or trait have been linked to appropriate medical care and all needed resources.

The Missouri SCDTDP grantee network worked to improve follow up after screening through the Parents As Teachers program, where they have a trained staff member educate parents of infants 0-3 years old. The program includes home visits, developmental assessment, answering parental questions and reminders about clinic appointments so that children receive all recommended care. The grantee has also conducted extensive outreach around genetic education in local schools.

The Tennessee SCDNBSP grantee network has worked to provide ongoing preconception hemoglobinopathy trait testing and education to at least 10 percent of the at-risk adult population of child-bearing age. To improve the education offered, providers, parents, and sickle cell experts worked together to develop a knowledge assessment tool that could be used with an educational video to increase awareness. The tool assesses knowledge of sickle cell trait, provides information on eight domains of knowledge that have been recommended for genetic counseling and includes a 24-question quiz (a fun way for adults to find out how much they know, or don't know, about sickle cell trait). The grantee reported strong levels of collaboration and communication streams among sites and partners within their network, which is promising for the spread, uptake, and sustainability of this knowledge tool for screening.

**TABLE 7:** Changes tested by grantee networks in screening and follow up

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
Illinois	<ul style="list-style-type: none"> <li>Trait screening follow up (parent education and pre/post test)</li> <li>Prompt in primary care electronic health record system at three patient ages for specific trait education and genetic counseling</li> <li>Patient passport</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Pre/post test allowed staff to improve their teaching and to re-explain to parent any concepts that remained unclear</li> <li>Willing primary care practice to trial the electronic health record tool</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>At first, post-counseling tests were missed until process mapping done of clinic flow and changes made to facilitate post-test completion</li> </ul>
Missouri	<ul style="list-style-type: none"> <li>Parents as Teachers program</li> <li>Free testing and genetic counseling</li> </ul>	
Pennsylvania (SCDTDP & SCDNBSP)	<ul style="list-style-type: none"> <li>Sickle cell trait follow-up phone counseling</li> </ul>	
Tennessee	<ul style="list-style-type: none"> <li>Group visits for newborns with sickle cell disease (medical &amp; educational)</li> <li>Sickle cell disease center for Western Tennessee in partnership with state newborn screening program</li> <li>100 percent education rate for families of newborns with sickle cell disease in catchment area</li> <li>Free treatment and care for all newborns with sickle cell disease</li> <li>&gt;75 percent education rate for newborns with sickle cell trait</li> <li>Free testing for infant/family offered for newborns with sickle cell disease and trait</li> <li>Multi-disciplinary meetings for newborns with sickle cell disease (medical and educational)</li> <li>Free genetic education for families of newborns with sickle cell disease</li> </ul>	
<b>SCDNBSP Grantees</b>		
Illinois	<ul style="list-style-type: none"> <li>Group visits for newborns with sickle cell disease (medical and educational)</li> <li>Trait screening follow up (parent education and pre/post test)</li> <li>Work with state department of health to ensure that newborns with sickle cell disease are connected with a hematologist</li> <li>Free testing/genetic counseling</li> </ul>	
Massachusetts	<ul style="list-style-type: none"> <li>Trait screening results entered into electronic health record in standardized way</li> <li>Developed trait education materials for primary care providers and parents</li> <li>Prompt in electronic health record to provide genetic counseling at 3 time points – infancy, to counsel parents about future pregnancies; school age and adolescence to educate child; available at Boston Medical Center and 3 community health centers</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Tracking use of prompt by provider has led to increased counseling</li> <li>Pediatric primary care providers are notified of upcoming visits to remind them to provide counseling; this work will be added to other initiatives (asthma, etc.), making it sustainable</li> <li>Education and regular reminders at provider meetings increase use of prompt</li> <li>Sickle cell champions at each community health center advocating for increased identification and counseling for sickle cell trait</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Difficult to standardize the identification and counseling to adults with sickle cell trait who did not have newborn screening through IT</li> <li>Tracking prompt use is currently labor intensive; need alternative funding to sustain</li> <li>Multiple competing priorities (including medical home certification) make it difficult to keep screening efforts on top of list</li> </ul>
New York	<ul style="list-style-type: none"> <li>Free testing/genetic counseling</li> <li>Screening checklist developed and tested</li> </ul>	
Ohio	<ul style="list-style-type: none"> <li>Business-sized reference card with hemoglobin type and highlights</li> <li>Free testing and genetic counseling</li> </ul>	
Tennessee	<ul style="list-style-type: none"> <li>Trait education and knowledge tool and video</li> <li>Knowledge survey about sickle cell disease</li> </ul>	



## Transition of Care

Many individuals with sickle cell disease do not experience a smooth transition from pediatric to adult care. They may not have adequate knowledge or enough practice managing their medications and appointments, and it may be difficult for them to find appropriate adult providers and health care coverage. As a result, mortality rates can be elevated for young adults making this transition.<sup>53</sup> A successful transition program can prepare young adults for this challenging time and help them avoid unnecessary complications of the disease. Grantee networks developed and tested many tools and resources to use with transitioning young adults. Some grantees saw improvement in process-level measures such as the percentage of adolescents given a transition readiness tool, but overall the program did not see improvement in the outcome measure, the percentage of patients with a written transition plan.

*“When I left the pediatric sickle cell team I was afraid because I was told that adult sickle cell patients are not treated as well but Dr. Alice and the Central Northern Sickle Cell Network has renewed my expectations and I can now say I am treated very well and people genuinely care about me and sickle cell disease.”*

– NJ SCDTDP patient

Because of great strides over the past few decades in care for individuals with sickle cell disease, these individuals are now living longer, transitioning from pediatric to adult care as they grow older. As patients transition from pediatric care to adult care, they experience a variety of challenges including leaving a familiar provider and environment, being seen by a provider who may not have knowledge of sickle cell disease, establishing independence from caregivers, and having adequate health insurance.<sup>54</sup> Multiple factors may contribute to high mortality during the period immediately following transition from pediatric to adult care including disease progression, lack of routine care and adherence to treatment.<sup>55</sup> In addition to increased mortality, young adults with sickle cell disease utilize emergency care services more often and have less frequent care maintenance visits during the transition years.<sup>44</sup> Planned and coordinated transition from pediatric care to adult care is critical in ensuring no interruption in care continuity and improving health outcomes and overall quality of life of individuals with sickle cell disease.

Nine grantees have worked to improve the process by which adolescents with sickle cell disease transition from pediatric to adult care through changes such as coordinating primary and specialty care, working with patients to ensure transition readiness, creating transition clinics and working across grantees to share successful change ideas and resources through the Transition Affinity Group. Since transitioning from adolescence to adulthood involves more than just transferring from pediatric to adult clinical care, grantees have worked to facilitate transitions for young adults in many other domains such as higher education, insurance, employment, and other psychosocial issues.

The high-leverage changes that grantees tested were:

- **Transition Clinic:** Many of the collaborative grantees have worked to develop or improve their pre-existing transition clinic or program. The purpose of these clinics is to provide a dedicated time to prepare adolescents to move from pediatric medical care to adult medical care. In an ideal setting, there is some overlap (or a gradual transition) between seeing pediatric providers and adult providers, and in some cases there is a “warm hand-off,” in which pediatric staff members accompany youth to adult visits. Grantees have improved readiness materials, provided information to patients in early adolescence, linked patients with adult providers and services before transfer of care is completed, and ensured that both the patient and family are ready for the transfer to adult care. These strategies align with the recommendations from the National Center for Healthcare Transition Improvement.<sup>56</sup>
- **Transition Readiness Assessment:** Several grantees have developed or adapted some form of transition readiness assessment based on the work of the Got Transition? National Center for Health Care Transition Improvement<sup>56</sup>, which allows adolescents and their health care providers to assess knowledge and self-efficacy among different domains of knowledge including medical, cognitive/emotional, psychosocial, and academic.

- **Sickle Cell Disease-Specific Transition Curriculum:** The Transition Curriculum was developed through a collaboration among the grantees in the Transition Affinity Group. The comprehensive curriculum covers all ages of the transition period (12-21 years of age) and includes recommendations of content for all providers, patients and parents to reference, which did not exist previously. The curriculum is divided into three main sections by age group, and each age group consists of three domains: Medical, Social, and Academic. Each domain includes guidelines for medical topics, methodology, and techniques to measure efficacy.

### Results in Transition of Care

Upon review of the aggregate data for the percent of patients with a written transition plan, very little improvement can be seen in this metric. This represents an opportunity for ongoing improvement efforts. Some grantees were able to see improvement in some process level measures. The Colorado SCDTDP grantee network was able to increase the percentage of adolescents given their transition readiness tool (Figure 13). The California SCDTDP grantee exceeded their goal of distributing their transition brochure to 50 percent of eligible patients (Figure 14), in keeping with the first core component of transition, educating patients and families about the transition policy.

**FIGURE 13:**

### Colorado SCDTDP: Percentage of adolescents given transition readiness tool

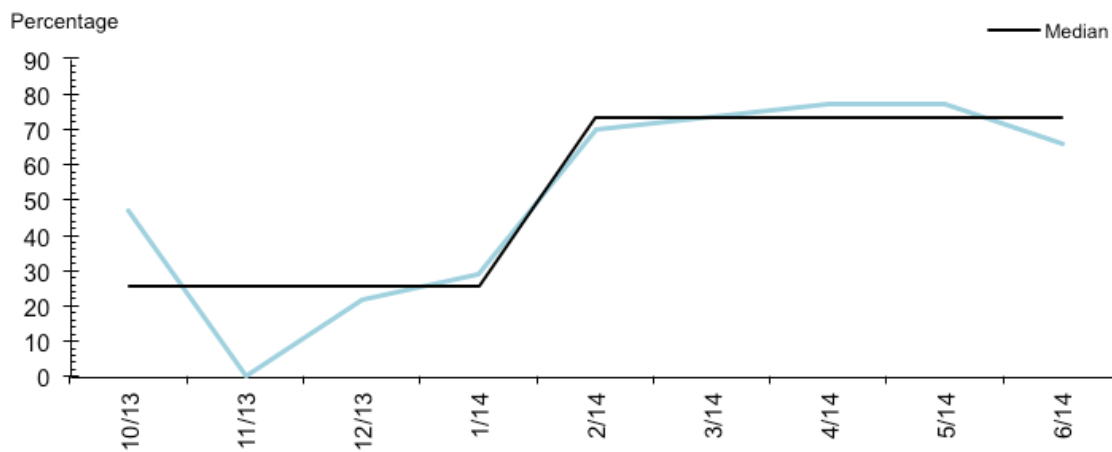


Figure 13. Run chart representing data from the CO SCDTDP team. The team was able to increase the percent of adolescents given their transition readiness tool to 70 percent.

**FIGURE 14:**

*California SCDTDP: Percentage of eligible patients receiving transition brochures*

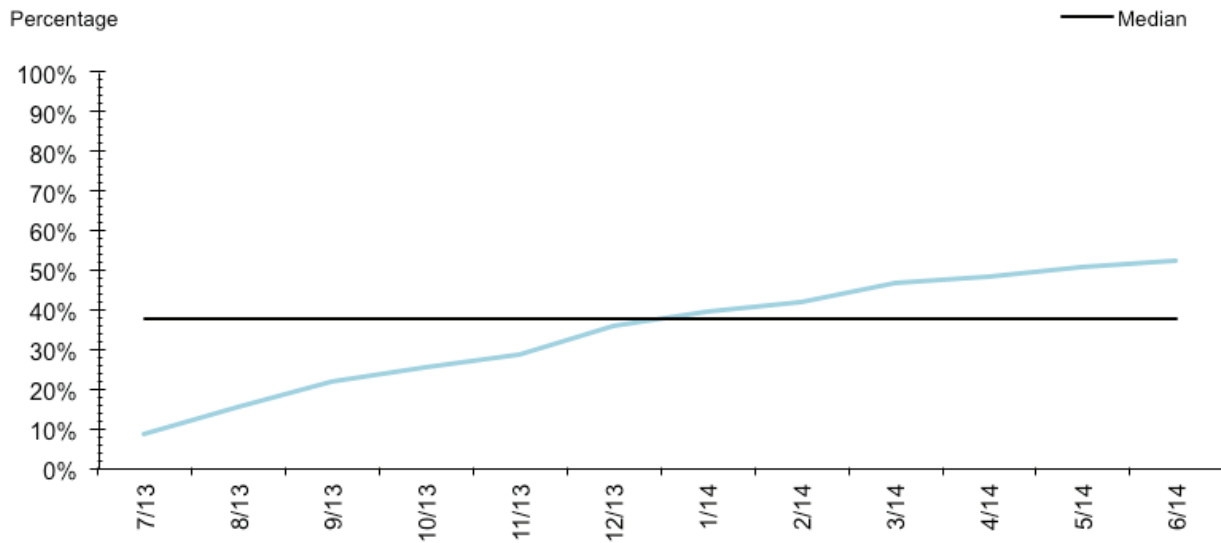


Figure 14. Run chart representing data from the CATDP team. CATDP was able to increase the percentage of eligible patients receiving a transition brochure to 52 percent (67 of 128 eligible patients) by June 2014, surpassing the goal of 50 percent.

*Highlights of Grantee Work in Transition of Care*

The Tennessee SCDTDP grantee network developed a transition clinic nearly three years ago with institutional support in which the pediatric hematologist sees patients in consultation with an adult internist. While the hematologist is only in clinic on certain days, the internist is present every day and is available to respond to primary care inquiries. A nurse practitioner also provides medical support. By bringing pediatric and adult providers together, the clinic enables patients to get to know an adult provider while still receiving most care from their pediatric care team. In addition to having the physical space for the clinic, the Tennessee team offers education materials to inform individuals about sickle cell disease, a series of web modules for transition preparation, and a text message reminder system that patients can use to manage medication adherence.<sup>57</sup>

*“Thank you for transitioning me and making me who I am today not just as a patient but a person. I thank my nurses, doctors, and teachers for teaching me more about my disease but also how to live a healthy life with it.”*

– TN SCDTDP patient

The Pennsylvania grantee network has worked on a transition program that is based at their network’s community-based organization partner, Children’s Sickle Cell Foundation, and partners with the Children’s Hospital of Pittsburgh. The transition clinic uses a patient-centered approach of “Transition Together” based on tenets from the Advanced Practice Model designed by a Family Nurse Practitioner,<sup>58</sup> and incorporates a wide variety of training in life skills as well as sickle cell disease-specific knowledge and academic support.

**TABLE 8:** Changes tested by grantee networks in transition

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
California	<ul style="list-style-type: none"> <li>• Transition policy</li> <li>• Transition clinic</li> <li>• Transition readiness assessment</li> <li>• Community-based transition workshop</li> <li>• Bridge staff to follow transitioning youth/young adults into sickle cell adult care</li> <li>• Transition satisfaction questionnaire</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Co-location of the adult and pediatric programs</li> <li>• Collaboration with Virginia Commonwealth University on Transition Intervention Program that it had already created</li> <li>• Very committed social worker who took the lead to test the best approaches to setting up transition clinics and other supports</li> <li>• Staff very committed to educating patients about the transition policy, once it was developed</li> <li>• A community-based organization puts in months of planning and brings together a range of community providers (e.g. teachers, a financial planner, and an internist) to educate youth and families at the annual transition workshop</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Patients do not have options about where they are transitioned to – the only adult program in the region is ours</li> <li>• Shift in state insurance requirements to require unknown primary care providers for each adult patient</li> <li>• Departure of family nurse practitioner</li> <li>• Attendance at community-based transition workshop very low</li> </ul>
Colorado	<ul style="list-style-type: none"> <li>• Transition clinic</li> <li>• Transition readiness assessment</li> <li>• Adoption of technology/mobile app for pain</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Clinical model existing at the University of Colorado School of Medicine for transition for cancer patients helped inform transition efforts for sickle cell disease patients</li> <li>• Faculty at the University of Colorado School of Medicine have been involved with the Got Transition program nationally</li> <li>• Grant funding allowed for dedicated time to allot for pediatric case manager to lead transition program planning and develop readiness efforts</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Small number of transitioning individuals each year makes planning any transition efforts resource intense, and hinders institutional buy-in to sustain efforts</li> <li>• Hard to contact transitioning individuals</li> <li>• Level of patient support/outreach in adult settings very different from pediatric settings (e.g., longer wait times in adult emergency department)</li> </ul>
Illinois	<ul style="list-style-type: none"> <li>• Community health workers assisted in the development of self-management skills</li> <li>• Written pain management plans for adult patients</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• For the readiness assessment, a one-page checklist made it easier to track what had been covered at each visit over time</li> <li>• Using already established materials saved us time from developing our own</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Small number of transitioning individuals each year makes planning any transition efforts resource intense, and hinders institutional buy-in to sustain efforts</li> <li>• Low numbers of transitioning patients per year slowed PI assessments</li> </ul>
Maryland	<ul style="list-style-type: none"> <li>• Transition support groups</li> <li>• “Transition nights” (educational programs with transition mentors who are adults with sickle cell disease)</li> <li>• Patient ambassadors (adult patients who work directly with patients to assist with transition)</li> <li>• Sickle cell fact sheets for adolescents</li> </ul>	
Missouri	<ul style="list-style-type: none"> <li>• Transition clinic</li> <li>• Transition readiness assessment</li> </ul>	

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
New Jersey	<ul style="list-style-type: none"> <li>Transition policy</li> <li>Education program for ages 13-18 with pre/post tests</li> <li>Transition education fairs</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>We have a weekly team meeting attended by pediatric and adult teams to discuss patients and transition process.</li> <li>The pediatric social worker has sickle cell disease.</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Parents sometimes reluctant to transition to adult service (due to fear of adult emergency department and inpatient care)</li> </ul>
Ohio	<ul style="list-style-type: none"> <li>First visit program: patient accompanied by pediatric team to first adult primary care provider visit</li> <li>Adult transition steering committee</li> <li>Developed concierge-style handbook to introduce adult medical campus</li> <li>Provided guided tours of adult campus</li> <li>Adult transition steering committee representation at monthly emergency department improvement meetings</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Hospital administration's focus on improving the transition process for all chronic illness groups</li> <li>Supportive hematology and primary care medical directors</li> </ul> <p><b>Barriers:</b></p> <ul style="list-style-type: none"> <li>Differences between pediatric and adult providers' approaches to co-management with primary care</li> </ul>
Pennsylvania (SCDTDP & SCDNBSP)	<ul style="list-style-type: none"> <li>Transition clinic at community-based organization</li> <li>Motivational interviewing</li> <li>Portable medical summary</li> </ul>	
Tennessee	<ul style="list-style-type: none"> <li>Transition curriculum</li> <li>Transition clinic</li> <li>Patient/provider transition readiness assessment</li> <li>Technology/texting reminders</li> <li>Transition tour with teens/parents/adult providers/adults with sickle cell disease /social/academic</li> <li>Transition database developed</li> <li>Transition booklet developed</li> <li>Web-based educational material developed</li> <li>Monthly transition assessment multidisciplinary meetings</li> <li>Follow up from pediatric to adult care by nurse case manager</li> <li>Virtual mentor program to pair young adult with sickle cell disease with teen with sickle cell disease</li> </ul>	
<b>SCDNBSP Grantees</b>		
Massachusetts	<ul style="list-style-type: none"> <li>Transition clinic</li> <li>Transition readiness assessment</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Pediatric and adult hematology providers participate</li> <li>Patient navigator sends visit reminders, assesses whether adult primary care has been established</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Lack of follow up, unsure if transition is successful since patients expected to assume responsibility for own care after transition</li> <li>Level of patient support/outreach in adult settings very different from pediatric settings</li> </ul>
New York	<ul style="list-style-type: none"> <li>Transition policy</li> <li>Transition curriculum</li> <li>Transition readiness tools</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Hemoglobinopathy Learning Collaborative</li> <li>Got Transition.org</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Small cohort</li> <li>Adult clinic infrastructure is new for sickle cell</li> </ul>

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDNBSP Grantees</b>		
Ohio	<ul style="list-style-type: none"> <li>• Transition clinic</li> <li>• Patient/provider goal setting</li> </ul>	<p><i>Facilitators</i></p> <ul style="list-style-type: none"> <li>• Monthly adolescent and young adult clinic</li> <li>• Monthly transition team meeting (pediatric and adult providers)</li> <li>• Institutional support for tracking transition metrics</li> <li>• Electronic health record support for documentation of transition readiness, progress and satisfaction survey</li> <li>• Transition coordinator</li> <li>• Built-in opportunities for transition education (e.g., camp, research day, etc.)</li> <li>• Institutional support for an adolescent and youth support and mentoring program</li> <li>• Good relationships between pediatric and adult hematology programs</li> </ul> <p><i>Barriers</i></p> <ul style="list-style-type: none"> <li>• Staff turnover</li> <li>• Differences in institution priorities (pediatric vs. adult)</li> <li>• Competing institutional priorities</li> </ul>

## Hydroxyurea

Hydroxyurea, the only approved therapy for sickle cell disease, can have a tremendous impact on patients' quality of life by reducing complications of the disease. However, use of hydroxyurea varies a great deal by provider and institution, and poor understanding of the drug and its side effects limits its use. Several grantee networks have been working on practice guidelines and educational materials focused on increasing the use of hydroxyurea.

Hydroxyurea is the only therapy approved for sickle cell disease by the Food and Drug Administration.<sup>8,59</sup> This medication results in a decline in sickle cell-related complications such as pain crises, acute chest syndrome and associated emergency department visits and hospitalizations.<sup>60</sup> By reducing the frequency of these complications of sickle cell disease, hydroxyurea can improve the quality of life for patients.<sup>9,61</sup> Hydroxyurea has been found to lower the costs associated with care for patients with sickle cell disease. While outpatient costs have been found to be higher, they are outweighed by the savings from fewer hospitalizations.<sup>60</sup>

The National Institutes for Health Consensus Development Conference: Hydroxyurea Treatment for Sickle Cell Disease was part of an ongoing program that produced unbiased, evidence-based assessments of controversial medical issues important to researchers, healthcare providers, policymakers, patients, and the general public. The statement resulting from this conference highlighted that hydroxyurea is underused.<sup>62</sup> The use of this disease modifying therapy varying by provider and institution highlights a substantial opportunity to improve sickle cell care by making hydroxyurea accessible to more patients.<sup>60,63,64</sup> Prior research on other chronic illnesses suggest that provider-patient communication may influence hydroxyurea use.<sup>65,66,67,68</sup> Barriers include incomplete understanding of the clinical benefits, side effects, and long-term consequences of its long term use among patients and providers.<sup>69,70</sup> Additional barriers to hydroxyurea use are also focused at the health system level (e.g., insurance coverage).

Grantees implemented different types of changes. Some worked with physician champions to make hydroxyurea an integral part of the care of sickle cell disease and increase the percentage of eligible patients who had a discussion about hydroxyurea with their provider. Two grantees (Illinois SCDNBSP and Ohio SCDNBSP) undertook a survey of patients, families and providers to further refine their improvement efforts.

The high-leverage changes that grantees tested were:

- **Improved provider education:** Nearly all grantees developed guidelines and training materials to facilitate education of providers.
- **Improved patient education:** Grantees created videos, brochures, and other information sources for patients and families. Grantees also educated providers to facilitate better communication between providers and patients about treatment with hydroxyurea.
- **Decision support tools:** Several grantees are developing shared decision making tools to guide patients and families through the process of evaluating hydroxyurea therapy.

### Aggregate Results in Hydroxyurea

Ten grantees have tracked the percentage of eligible patients who are on hydroxyurea at their site. These data are collected quarterly. Upon review of these data, the percentage of patients currently using hydroxyurea has been stagnant over time (Figure 15). These data were only reported for four total quarters, which represents a short time horizon to make significant improvements in this measure. It is important to note, however, that several grantees do have more than 50 percent of their patients on hydroxyurea; the aggregate data reflect the average across all grantees. Sickle cell patients' decisions to take hydroxyurea are a complex interplay of several factors including their preferences, their understanding about the therapy, potential benefits and side effects, patient and provider communication, provider awareness about therapies, and provider endorsement or lack of endorsement of the therapy. Several of these factors may explain why there was not significant increase in the percentage of hydroxyurea use across all collaborative sites in this timeframe.

**FIGURE 15:**

### Percentage of eligible patients taking hydroxyurea (Hemecare 5)

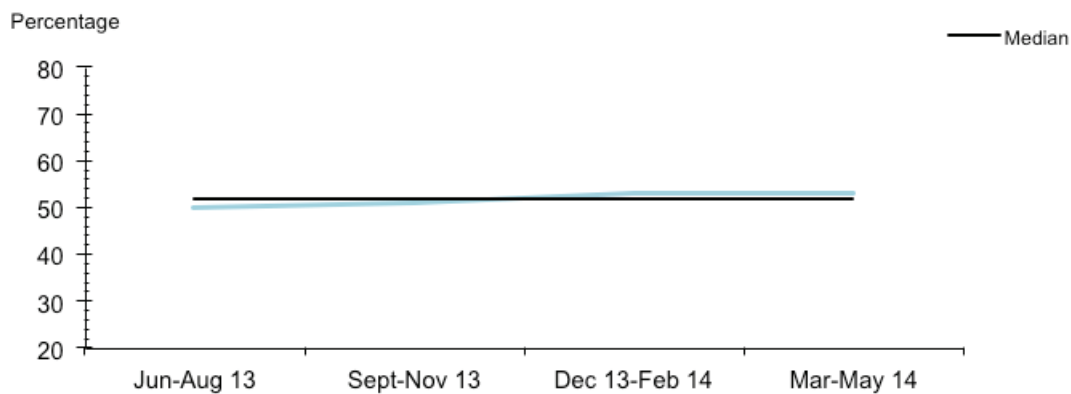


Figure 15. Run chart representing data from 10 teams. The collaborative median was reported as 50 percent during the first quarter and moved to a total of 53 percent in the final reporting quarter.

### Highlights of Grantee Work in Hydroxyurea

The New Jersey SCDTDP grantee network has worked extensively on improving the number of eligible patients in their network on hydroxyurea, which they started to identify by developing a registry of eligible patients. They worked to increase patient knowledge about hydroxyurea through videos and education by providers and also developed a dose management tool for hydroxyurea to ensure optimal dosing and outcomes for patients.

The Massachusetts SCDNBSP grantee network revised their guidelines for hydroxyurea use in May 2012, expanding eligibility to include all children with more severe sickle cell genotypes. The pediatric hematologists began discussions with all eligible children and their families, discussing the benefits with respect to decreased morbidity and improved long-term survival, based on available data from adult studies. To further support educational efforts, they created an interactive educational module to assess patients'/guardians' knowledge, beliefs and attitudes on hydroxyurea and to educate them about the role of hydroxyurea in sickle cell disease. Of the 20 families who completed the education module with a provider, 13 initiated treatment with hydroxyurea, five are still considering it, and two declined. From June 2012 through June 2014, MA SCDNBSP increased the number of children on hydroxyurea from 50 percent to 79 percent (Figure 16).

*"I think since a lot of people are not used to it [hydroxyurea] and they hear that it is used for cancer they get afraid. My daughter was always in the hospital and I just watched her suffer and I couldn't do anything about it. But, with hydroxyurea, she has just been taking it for one month and I have seen drastic improvement. She is happier, she is running around and she is much healthier"*

– MA NSBP parent

**FIGURE 16:**

### Massachusetts SCDNBSP: Percentage of eligible patients on hydroxyurea (Hemecare 5)

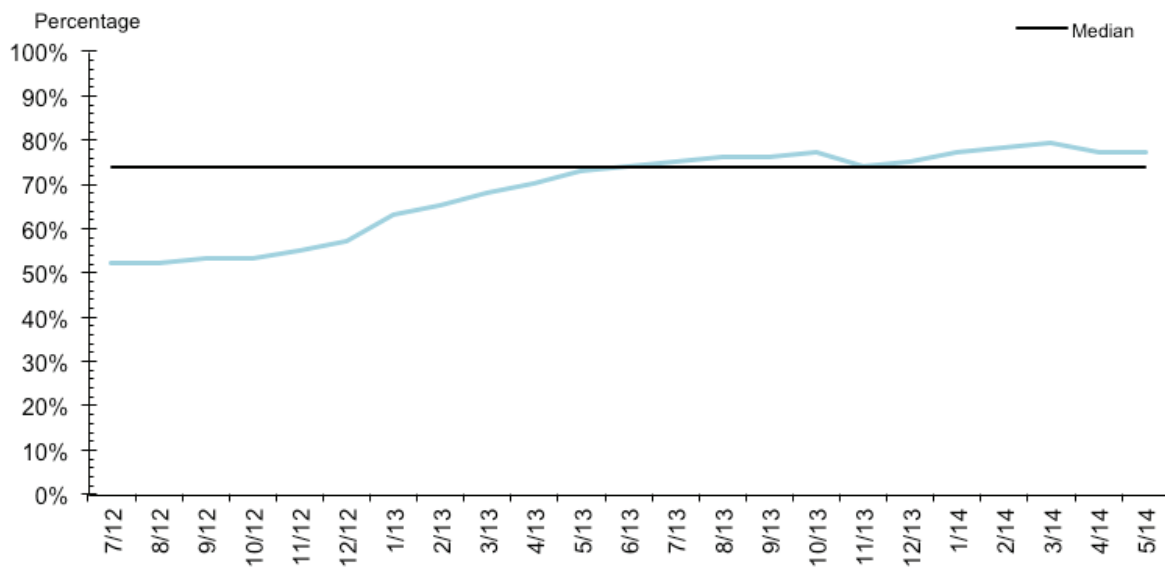


Figure 16. Run chart representing data from MA SCDNBSP team. The MA team was able to increase the percent of eligible patients on hydroxyurea from 50 percent to 75 percent.



**TABLE 9:** Changes tested by grantee networks in hydroxyurea

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
Illinois	<ul style="list-style-type: none"> <li>Educational materials</li> </ul>	<p><i>Facilitators</i></p> <ul style="list-style-type: none"> <li>Could draw from available resources</li> </ul> <p><i>Barriers</i></p> <ul style="list-style-type: none"> <li>Not readily available in foreign languages</li> </ul>
New Jersey	<ul style="list-style-type: none"> <li>Education materials including video</li> <li>Registry of eligible patients</li> <li>Hydroxyurea tracking form for dose escalation</li> </ul>	<p><i>Barriers</i></p> <ul style="list-style-type: none"> <li>Fear of toxicity that appears in package insert, online information about hydroxyurea when used for other diseases</li> </ul>
Ohio	<ul style="list-style-type: none"> <li>Electronic health record tool</li> <li>Patient level baseline review of hydroxyurea use by pharmacists</li> <li>Pharmacist to provider consults regarding individual patient's adoption of hydroxyurea</li> <li>Pharmacist reviews with patients regarding adherence and appropriate medications</li> </ul>	<p><i>Facilitators</i></p> <ul style="list-style-type: none"> <li>Two pharmacists plus their rotating trainees stationed one session a week in the sickle cell disease clinic</li> <li>Pharmacist participation in weekly care coordination meeting</li> </ul> <p><i>Barriers:</i></p> <ul style="list-style-type: none"> <li>Transitions in the medical director of the sickle cell disease clinic made finalizing a standard approach to hydroxyurea difficult</li> </ul>
Pennsylvania (SCDTDP & SCDNBSP)	<ul style="list-style-type: none"> <li>Shared decision making tool</li> <li>Telemedicine</li> <li>Consistent messaging</li> <li>Direct observation therapy</li> </ul>	
Tennessee	<ul style="list-style-type: none"> <li>SIMON program (program to increase compliance and communications about hydroxyurea)</li> <li>Protocol change to offer hydroxyurea to younger children with sickle cell disease</li> <li>Hydroxyurea educational booklet</li> <li>Protocol change to educate parents at first visit about hydroxyurea</li> </ul>	
<b>SCDNBSP Grantees</b>		
Illinois	<ul style="list-style-type: none"> <li>Patient survey around knowledge and decision-making</li> </ul>	
Massachusetts	<ul style="list-style-type: none"> <li>Individual education, development of electronic and written materials</li> <li>Revision of electronic health record to easily track hydroxyurea education and adherence</li> <li>Registry used to track use of hydroxyurea through monthly reports</li> <li>Developed hydroxyurea guidelines for pediatric patients that expanded eligibility to all those with HbSS/Sβ<sup>0</sup> who are ≥ 1 year old.</li> </ul>	<p><i>Facilitators</i></p> <ul style="list-style-type: none"> <li>Tracking hydroxyurea use in registry/monthly reports for pediatric patients, including date of last prescription written.</li> </ul> <p><i>Barriers</i></p> <ul style="list-style-type: none"> <li>Approximately 20 percent of eligible children not receiving hydroxyurea, perception that they are “well” by parents</li> <li>Clinic notes in adult hematology not standardized, difficult to track hydroxyurea use, adherence</li> </ul>
New York	<ul style="list-style-type: none"> <li>Increased patient education at younger age/support group topic/parent peer education utilized</li> </ul>	<p><i>Facilitators</i></p> <ul style="list-style-type: none"> <li>Efficacy of the medication and the experience of our patients already on it.</li> </ul> <p><i>Barriers</i></p> <ul style="list-style-type: none"> <li>General medication and appointment compliance issues for a few patients</li> </ul>

Grantee	Intervention & Aspects of Care Addressed	Facilitators of & Barriers to Change
<b>SCDNBSP Grantees</b>		
Ohio	<ul style="list-style-type: none"> <li>Shared decision making tool</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Quality improvement team who meets regularly</li> <li>3 physician champions</li> <li>Engaged parents</li> <li>SCDTDP and SCDNBSP partners</li> <li>Design students from the University of Cincinnati</li> <li>Multidisciplinary team with expertise in shared-decision making, qualitative research methods and medication safety</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Patient no-shows</li> <li>Changes in clinic flow process</li> <li>Staff turnover</li> <li>Multiple sites</li> </ul>
Tennessee	<ul style="list-style-type: none"> <li>Education</li> </ul>	

## Outcome Measures

All grantees were asked to report data on emergency department utilization and hospitalizations irrespective of the focus area of their efforts. Because grantees' work in all five core areas described above could potentially influence the frequency of emergency department visits and hospital admissions, these measures served as collaborative-wide outcomes. Grantees were asked to report these data quarterly for a consistent sample of their patient population. The sample size varied across teams, but was usually at least 80 percent of the patient population or no more than 100 patients.

Based on these data, we are able to aggregate total emergency department visits and hospitalizations per month as well as the average proportion of the population who were experiencing at least one emergency department visit or hospitalization for sickle cell pain. The data on the proportion of the population utilizing services proves more sensitive to the overall collaborative work, since the sample size per site varied greatly.

These data were collected between June 2013 and May 2014, the last year of the project period. Delays in starting this data collection were largely due to the length of time needed to gain both Institutional Review Board and Office of Management and Budget approval. Seeing improvement in these measures so far into the project period is difficult as grantees were making changes to their systems long before these data were collected and may have already realized improvement.

### Emergency Department Visits

Although there are fluctuations in the percentage of patients with at least one emergency department visit, no significant reductions over time can be observed (see Figure 17). The median is 9.75 percent. Overall, this is a small percentage of patients who are seeking acute care services for sickle cell pain which suggests that patients may be more effectively managing their pain episodes at home and/or potentially seeking care at day hospitals rather than going to the emergency department.

**FIGURE 17:**

*Percentage of patients with at least one emergency department visit for sickle cell disease-related pain in the last month (represents ~900 patients each month)*

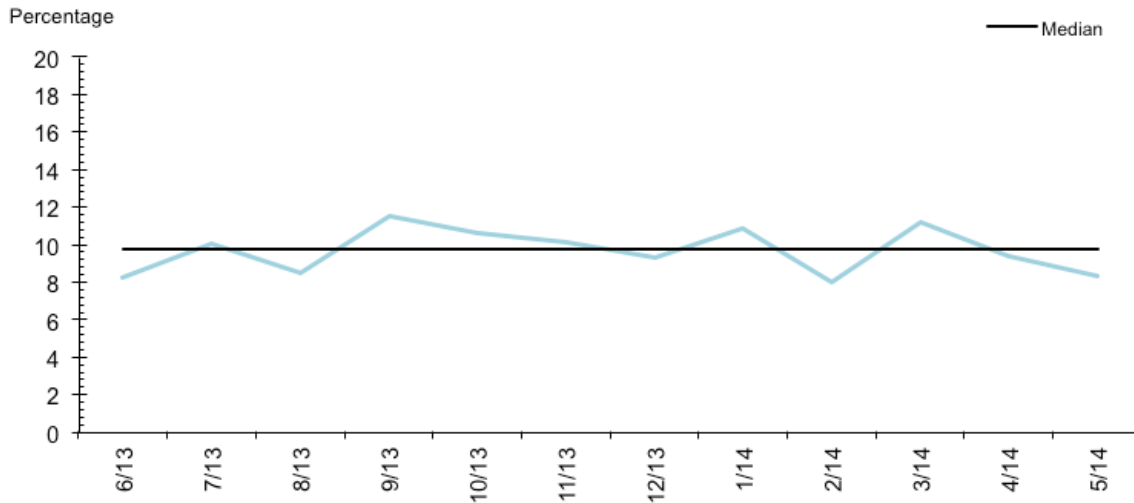


Figure 17. Run chart representing data from 10 grantees. The median is 9.75 percent, and no significant changes can be observed. This is a median across all patients at all sites.

### *Hospitalizations*

In reviewing the percent of patients with at least one hospitalization from June 2013 through May 2014, we see a slight improvement over time with five consecutive data points showing a reduction in this measure from July 2013 to December 2013 (Figure 18). This is a sign of non-random variation, indicating that grantees' improvement efforts appear to have reduced the overall percentage of patients with at least one hospitalization from July 2013 to December 2013, moving from 10.2 percent to 8.4 percent. This finding may be due to increased uptake of effective therapies such as hydroxyurea among patients at the network sites, and it may also be due to better ambulatory management of sickle cell-related complications such as pain and improvements in overall patient self-management of this condition. This gain was not sustained.

**FIGURE 18:**

*Percentage of patients with at least one hospitalization for sickle cell disease-related pain in the past month (represents ~900 patients each month)*

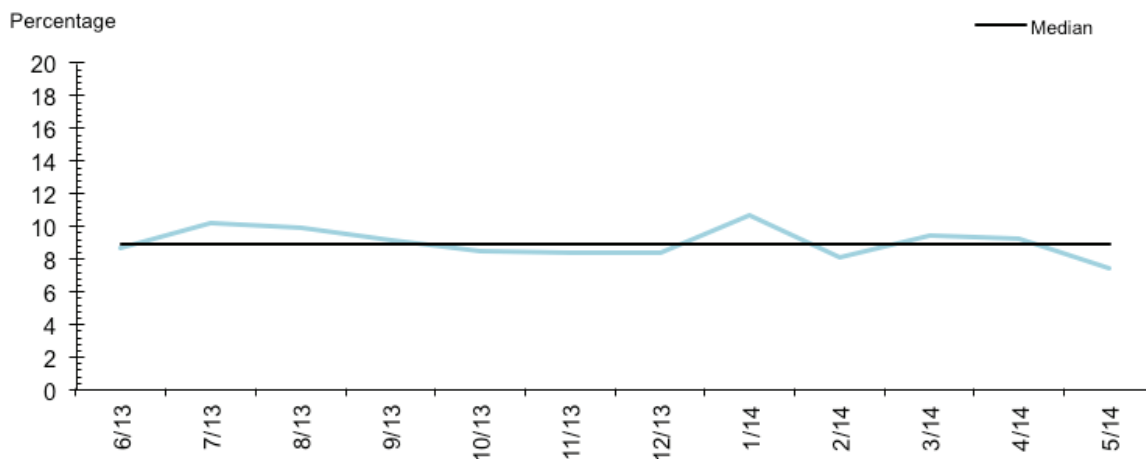


Figure 18. Run chart representing data from 10 grantees. Non-random variation can be observed in the five consecutive data points from July to December 2013, which show a decrease in this measure.

## RESULTS FROM OTHER GRANTEE ACTIVITIES

Grantees also worked on several other initiatives in addition to their quality improvement work. Their work with sickle cell disease partners and on education and outreach is described below.

### Outreach and Education/Increasing Awareness of Sickle Cell Disease

All of the grantees have performed outreach and education with providers, patients and families and the community in order to increase awareness and knowledge of sickle cell disease and sickle cell trait. Events ranged from health fairs to classroom visits, to collaborating with other organizations and performing free screening for sickle cell disease and sickle cell trait. Most education and outreach efforts focused on three audiences:

- **Providers:** Grantees held educational programs with a variety of providers, including emergency department staff, primary care providers, hematologists, nurses and patient navigators. Training for health professionals is vital to ensuring that health professionals are able to provide current medical care and appropriate support services to patients with sickle cell disease. Some educational programs were also attended by teachers and day care providers.
- **Individuals with sickle cell disease:** Grantees offered several events for individuals with sickle cell disease and their families. Events ranged from educational programs aimed at improving knowledge and management of sickle cell disease to support and social events that allowed individuals with sickle cell disease and their families to meet and learn from others with the disease.
- **General Public:** Grantees participated in many community events including health fairs, races, and church events to raise awareness about sickle cell disease and sickle cell trait.

### Highlights of Grantee Work in Education and Outreach

The Ohio SCDTDP grantee network supported the formation of an adult patient-run advocacy group: “S.CELL” (a Supportive Community of Educators Living Life). The vision of the group is “to be educators and advocates for the sickle cell community.” Their mission is to foster hope for patients and families affected by sickle cell disease through education; advocate for people with sickle cell disease so they can better receive support and care from their community; and encourage people with sickle cell disease to live healthy and productive lives. Seven consumers in S.CELL

have participated in 19 events and given 290 sickle cell quizzes to their community.

The California SCDTDP grantee network's outreach efforts reached 3,246 individuals at health fairs, blood drives, the sickle cell support group and other health-focused events. The team presented about sickle cell disease and the importance of minority blood donations at a professional baseball game that was attended by 20,000 people. The team also upgraded the California Sickle Cell Resources website ([www.casicklecell.org](http://www.casicklecell.org)) leading to a 62 percent increase in the number of visits to the website, with 662 unique from the U.S. and many other countries. Through this site, education to providers reached over 30,000 physicians, nurses, social workers, scientists and public health professionals locally, nationally and internationally.

*"He put together a bowling night for us and our families. It was so much fun to feel normal even if it's for one night. It also felt good to be around people that love us and understand us. With this disease you get sick so often that the nurses and doctors become your extended family. Haywood also put together a spa treatment for us. It was great. As a young woman with sickle cell disease my main focus is my illness so it was nice to relax and ask the doctors questions pertaining to other health topics."*

– NJ SCDTDP patient

*"This support group has provided sickle cell sufferers with enlightenment, education and has become a safe haven for sickle cell patients to express ourselves openly and freely about our ideas, fears, concerns and problems. Whether we need to discuss issues with medications, doctors, pain or just vent our emotions, Sandra and the others are always there to lend a hand, an ear to listen and sometimes a shoulder to cry on and when you have sickle cell, you need these things very often. In the past couple of months, Sandra, Haywood and the Sickle Cell Support Group and Wealth for Health Organization has provided the sickle cell patients like myself with nutrition classes, educational seminars, open forums with doctors and fun outings and trip for us and our family members. I am so very grateful for all that Sandra and the others of the Sickle Cell Support Group has done for us and the Sickle Cell Community of Jersey City. Please continue to keep up the good work."*

-NJ SCDTDP patient

In Colorado, a new community support group was started by a partnering community-based organization in metropolitan area outside of Denver. A local organization also partnered with a pediatric sickle cell case manager to host an artist's event, "Sickle Cell Art Creation Day" specifically targeted for youth in transition.

The Pennsylvania grantee network collaborated with Pittsburgh Public Schools and other school districts to create a network of advocacy and support geared toward achieving academic success for children with

*"My daughter had a sickle cell-related complication that required her to have a surgery. She had to stay at the Children's Institute for over two months. The Education Support Team helped her to maintain her school work."*

– PA SCDTDP/NBSP parent

sickle cell disease. An educational liaison worked with providers, families and schools to identify and address barriers that may prevent a child from achieving his or her academic potential such as absences, physical tiredness, lack of understanding of sickle cell disease and learning and cognitive difficulties. Over the past four years there has been an increase in the average GPA of the 191 children enrolled in the educational support program, from 2.02 to 2.93 with incremental increases each year (Figure 19). In the same period there has also been a steady decrease among these children in the average number of school absences per year, from 31 to 14 days (Figure 20). These data suggest the value of a comprehensive educational support and advocacy program for children with sickle cell disease.

**FIGURE 19:**

*Pennsylvania SCDTDP/SCDNBSP: Average GPA of children in the educational support program*

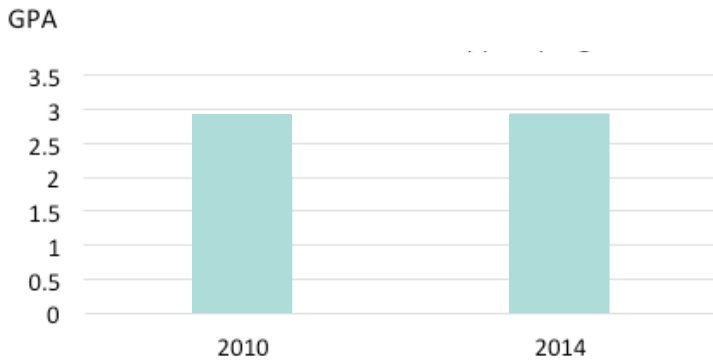


Figure 19: Average GPA for children enrolled in the Sickle Cell Children's Foundation's educational support program. Data from 191 children.

**FIGURE 20:**

*Pennsylvania SCDTDP/SCDNBSP: Average number of days per school year that children in the educational support program were absent from school*

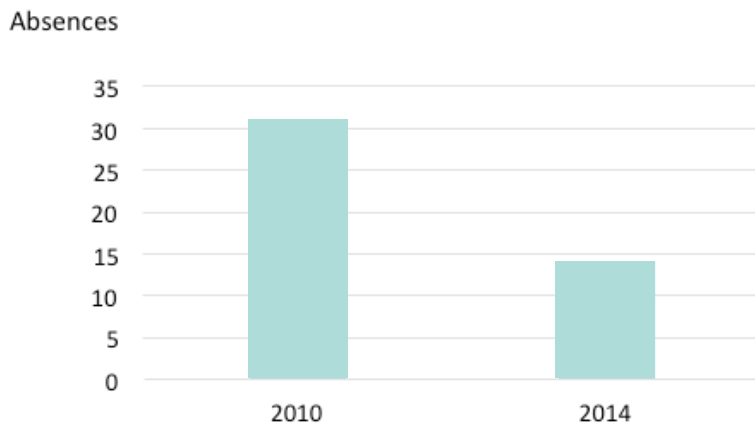


Figure 20: Average number of days per school year that children enrolled in the Sickle Cell Children's Foundation's educational support program were absent from school. Data from 191 children.

**TABLE 10:** Education and outreach events held by grantee networks

Grantee	Types of Events Held	Individuals Affected	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>			
California	<ul style="list-style-type: none"> <li>• Health fairs and blood drives</li> <li>• Sickle cell support group</li> <li>• Presented at a professional baseball game that was attended by 20,000 people</li> <li>• Upgraded California sickle cell resources website (<a href="http://www.casicklecell.org">www.casicklecell.org</a>) and Facebook page</li> <li>• Presented work at many national meetings</li> <li>• Sponsored advanced sickle cell disease workshop for clinicians from all over treatment of Health Care Services</li> </ul>	3,246	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Outreach arm of the sickle cell center already existed</li> <li>• Nationally and internationally known sickle cell disease experts (both research and clinical)</li> <li>• Strong community partners who collaborated on outreach including a sickle cell disease community-based organization, a blood center and Family Voices of California</li> <li>• Grant funding allowed website upgrade</li> <li>• Collaboration with the Centers for Disease Control and Prevention-funded Public Health Epidemiology and Surveillance for Hemoglobinopathies program on health promotion</li> <li>• Alignment with other statewide advocacy initiatives for individuals with special health care needs</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Attendance at support group meetings not optimal</li> <li>• Limited staff and funds for event planning and outreach</li> <li>• Attempting to partner with other community groups can be challenging if competing agendas surface</li> </ul>
Colorado	<ul style="list-style-type: none"> <li>• Development of professional-grade educational graphics on items used at home (e.g., thermometer, refrigerator magnet, key fob) to support families with low literacy</li> <li>• Materials developed by caregivers for child with sickle cell disease</li> <li>• Sickle cell disease screening booths held at community fairs and other local community events</li> <li>• Support and focus groups, held and run by partnering community-based organizations</li> <li>• Numerous events for patients, such as Annual Sickle Cell Disease Symposium and Sickle Cell Art Creation Day</li> <li>• Presentation at state-wide meetings</li> </ul>	~300 total (includes approx. 25 primary care providers total)	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Care for sickle cell disease in region was fairly well centralized</li> <li>• Setting in an academic medical center whose faculty members are knowledgeable about statewide health policy and other important initiatives</li> <li>• Goals of sickle cell center in line with overall mission of host academic medical center</li> <li>• Good working relationships between leadership team and those involved in public health activities and national activities such as transition</li> <li>• Leadership team includes both specialty and primary care providers</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Education, particularly of primary care and non-hematology specialists</li> <li>• Slight change in focus over course of the grant to quality improvement with a priori outcomes measures and focus on care coordination took priority in latter half of grant funding period</li> <li>• Relatively low prevalence and density of sickle cell disease in our region compared to other sites in the SCDTDP made need to advocate for sickle cell activities even more important</li> </ul>
Illinois	<ul style="list-style-type: none"> <li>• Health fairs</li> <li>• Sickle cell teen support group</li> <li>• Educational sessions to emergency department medical staff, medical students, rural federally qualified health centers, and rural hospitals via Grand rounds</li> </ul>	~400	
Maryland	<ul style="list-style-type: none"> <li>• Attended community events</li> <li>• Assisted in the organization of an annual sickle cell disease legislative day</li> <li>• Held “Urban Health” radio talks</li> <li>• Held events for community physicians offering continuing medical education credits</li> <li>• Conducted survey of primary care providers on comfort managing sickle cell disease patients</li> <li>• Created webinars on sickle cell disease management</li> <li>• Educational outreach through social media (Facebook and Twitter)</li> <li>• Community forums</li> <li>• Provided support to local sickle cell disease community-based organizations</li> </ul>		

Grantee	Types of Events Held	Individuals Affected	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>			
New Jersey	<ul style="list-style-type: none"> <li>• Adult and patient and family support groups</li> <li>• Formal education/lecture programs including education about self-management: Take Control of Your Health, transition workshops, and school lectures</li> <li>• Social education programs including Bowling for Awareness, Skating for a Cause, Eating Well with Sickle Cell</li> <li>• Attendance and informational table at numerous community health fairs, community races, and church events</li> </ul>	867	<p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Federally qualified health center's frequent turnover of physicians, nurses and executives' and lack of knowledge about the SCDTDP because of this turnover</li> </ul>
Ohio	<ul style="list-style-type: none"> <li>• Annual educational sessions with emergency department staff members</li> <li>• Session with entire 2<sup>nd</sup> year class of medical students about sickle cell disease</li> <li>• Educational sessions for community health workers</li> <li>• Health fairs and tables at church and other community events</li> <li>• Presentations to federally qualified health centers and other organizations</li> </ul>		<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Strong core of engaged patients with sickle cell disease</li> <li>• Network staff had previous experience engaging with consumer partners</li> <li>• The network's strong relationships with community based partners in the arenas of health and social justice provided access to public venues for S.CELL to provide education</li> <li>• Network staff's passion for sickle cell disease led to volunteerism to work events and facilitate team meetings outside of regular business hours</li> <li>• Active participation by patients in educational sessions for health professionals was well received</li> </ul> <p><b>Barriers:</b></p> <ul style="list-style-type: none"> <li>• Finding convenient timing for health professions' educational sessions</li> <li>• Some events cancelled due to very cold temperatures</li> </ul>
<b>SCDNBSP Grantees</b>			
Massachusetts	<ul style="list-style-type: none"> <li>• Grand rounds to internal medicine and family medicine departments</li> <li>• 8 presentations at pediatric primary care and adolescent practice group meetings</li> <li>• Presentations done at 4 community health centers</li> <li>• Health fairs at community events</li> <li>• Annual "Sickle Celebration" Walk and multicultural festival</li> <li>• Annual sickle cell disease conference</li> <li>• Community-based group sickle cell trait counseling presentation</li> <li>• Facebook page for Boston Medical Center pediatric sickle cell disease program</li> <li>• Boston City Council named September 15th as Sickle Cell Awareness Day</li> <li>• Appeared on 4 local TV and 15 radio programs to discuss sickle cell disease and trait</li> </ul>	1290	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Grant team, hematology providers, and community-based organization worked well together to do outreach and avoid duplication</li> <li>• Adult primary care provider champions identified, facilitated activities for adult providers</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Certain activities grant-funded (e.g., sickle cell trait counseling by community-based organization)</li> <li>• Limited availability of staff for community events limited outreach at times</li> <li>• Small number of sickle cell disease patients involved; hard to enlist their assistance and keep them engaged</li> </ul>
New York	<ul style="list-style-type: none"> <li>• Grand rounds to providers</li> <li>• Education to foster parents</li> <li>• Health fairs with onsite sickledex testing (a way of testing for sickle cell disease type)</li> <li>• Developed website</li> </ul>		<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>• Grant funds used to obtain translations Spanish/French for handouts</li> <li>• Networking led to participation in more health fairs and outreach than we could create ourselves.</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>• Limited staff and funds for event planning and outreach slowed the process.</li> </ul>



## Work with Sickle Cell Disease Partners

Sickle cell disease partners were vital team members who helped to inform and carry out the work within each of the networks. Engagement of partners included review and development of materials, testing of tools, outreach and training of physicians, nurses, medical students, and members of the community, and brainstorming ideas for self-management support.

### Highlights of Grantees' Work with Sickle Cell Disease Partners

The California SCDTDP grantee network's community partner, the Northern California Sickle Cell Community Advisory Council, collaborated in the implementation of quality improvement initiatives to improve self-management and acute care. The chair of the advisory council became a master trainer for Stanford University's Chronic Disease Self-Management Program and co-led two of those workshops for patients. Advisory council members participated in the Stanford University program and received additional training to serve as peer health coaches for the self-management project. An advisory council member participated in the grantee's monthly emergency department quality improvement project meetings, and the advisory council provided input on proposed approaches to improve self-management and acute care in sickle cell disease, including educational materials for families and providers.

*"As a patient and a member of the Sickle cell committee I have seen and have had firsthand experience of the growth that has occurred due to the Central Northern NJ. sickle cell network. One of the most important lessons is how to advocate for ourselves and how to take care of ourselves the best way possible. I can also speak my mind and share my ideas and I am being heard."*

– NJ NSBP patient

**TABLE 11:** Examples of sickle cell disease partner engagement

Grantee	Description of Engagement	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
California	<ul style="list-style-type: none"> <li>Led two Stanford University Chronic Disease Self-Management Program groups for patients</li> <li>Participated in emergency department quality improvement project meetings</li> <li>Held workshops on career and academic planning</li> <li>Education and in-service trainings to health care providers</li> <li>Initiated a community event to increase minority blood donors and to increase awareness about sickle cell disease</li> <li>Funded sickle cell center community health worker who was very active in sickle cell disease community-based organization and in statewide and national advocacy</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Highly motivated sickle cell disease community-based organization members and other community partners, such as Family Voices and regional blood center</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Some members of larger sickle cell disease community interested in starting their own organizations rather than collaborating with existing community-based organizations (resulting in a dilution of resources and energy)</li> </ul>
Colorado	<ul style="list-style-type: none"> <li>Sickle cell disease screening booths, held at community fairs and other local community events held and staffed by community-based organization partners</li> <li>Support and focus groups, held and run by community-based organization partners</li> <li>New support groups started in Colorado Springs</li> <li>Helped to host/lead the Sickle Cell Art Creation Day</li> <li>Partnered with pediatric sickle cell case manager to develop transition program and educational curriculum around transition</li> <li>Some patient navigators were also members of community-based organizations</li> <li>Sickle cell partners actively participated at all of the SCDTDP learning sessions</li> <li>Colorado Springs sickle cell disease partner lead helped conduct interviews in that metropolitan area</li> <li>Developed professional-grade educational tools for fever triage for families with low literacy</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Sickle cell disease community partners interested in and motivated to help those affected by sickle cell disease in their communities</li> <li>Members of Denver-based sickle cell disease partner group are bilingual Spanish-English speaking</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Sickle cell disease partner groups work in non-academic environment for the most part, creating need for subcontracts to help fund the partnership</li> <li>Competing demands/priorities in both environments slightly different—took time to better understand organizational priorities, processes and competing demands in different environments</li> <li>Strategies required to properly engage navigators and community partners when considering HIPAA issues and confidentiality</li> </ul>

Grantee	Description of Engagement	Facilitators of & Barriers to Change
<b>SCDTDP Grantees</b>		
Illinois	<ul style="list-style-type: none"> <li>Sickle cell disease partners were members of the advisory board</li> <li>Conducted educational outreach</li> <li>Leaders in self-management training</li> <li>Provided informal feedback on educational materials and teaching sessions</li> </ul>	<p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Time, transportation, child care were barriers for some patients and families</li> </ul>
Maryland	<ul style="list-style-type: none"> <li>Sickle cell disease patients and family members served on oversight committee</li> <li>Local sickle cell community-based organizations served on oversight committee and aided in project planning</li> <li>Provided financial support to local community-based organization activities</li> </ul>	
New Jersey	<ul style="list-style-type: none"> <li>Education and in-service trainings for health care providers</li> <li>Community health worker training for self-management program</li> <li>Participation in educational programs coordinated by community-based organizations</li> <li>Participation in community health fairs in network partners catchment area</li> <li>Educational programs for insurance carriers</li> <li>Emergency department algorithm created and shared with partner hospitals</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Partnerships with community-based organizations on educational programs and support groups including Sickle Celebration and self-management courses</li> <li>Medical Advisory Board attended by all partners in the network</li> <li>Support for blood drive in African American community coordinated by community-based organization</li> </ul>
Ohio	<ul style="list-style-type: none"> <li>Health educator with sickle cell disease employed during portion of project period</li> <li>Transition in sickle cell disease community-based organization during project period to newly formed organization comprised of adult patients with sickle cell disease</li> <li>Mentorship of new organization of sickle cell disease patients and family members, S.CELL, by network team</li> <li>Participation by S.CELL in health professions education sessions and community events</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Partnership with organizations not solely dedicated to sickle cell disease but serving the African American community, such as the Urban League and Be the Match</li> <li>Project director with expertise in community engagement provided needed support and facilitation of emerging advocacy group</li> </ul> <p><b>Barriers:</b></p> <ul style="list-style-type: none"> <li>No large established organization specific to sickle cell disease</li> <li>Limitation of consumer participation due to chronic illness and demands of daily life</li> </ul>
Pennsylvania (SCDTDP & SCDNBSP)	<ul style="list-style-type: none"> <li>Partners were members of the executive and coordination meetings held each month</li> <li>Partners were engaged in planning, implementation and evaluation of work in acute care, transition and care coordination, including toolkits for families of newborns and transitioning adolescents and the development of the new transition center</li> <li>Partners conducted and participated in educational outreach at churches, community events, schools, social service agencies and county agencies (e.g. including presentations to school administrators, teachers and students)</li> <li>Partners led trainings for health care providers such as physicians, nursing school students, and medical school students</li> <li>Partners participated in project activities such as affinity groups and learning sessions</li> </ul>	<p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Technology in data sharing, information transfer and the steep learning curve with data capture in a new system</li> <li>Not all parts of the network had access to compatible databases due to HIPAA and other limitations</li> <li>Initial difficulty with referral process, but managed to solve this with increased communication and secure emails</li> </ul>
<b>SCDNBSP Grantees</b>		
Massachusetts	<ul style="list-style-type: none"> <li>Pediatric patient navigator was a parent of a child with sickle cell disease</li> <li>5 parents and patients served on advisory board</li> <li>Participated in support groups and other events held by community-based organizations</li> <li>Approximately 50 patients/families with sickle cell disease from Connecticut and Massachusetts attended annual conference on sickle cell disease</li> <li>Participated in educational sessions hosted by community-based organization</li> <li>Parent was member of pediatric emergency department quality improvement team</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Committed core of volunteers reliably participated</li> <li>Strong support of community-based organization – helped identify potential consumers for grant and community activities</li> </ul> <p><b>Barriers:</b></p> <ul style="list-style-type: none"> <li>Limitation of greater consumer participation due to chronic illness, lack of transportation and child care</li> <li>Limited engagement of immigrant community</li> </ul>

Grantee	Description of Engagement	Facilitators of & Barriers to Change
<b>SCDNBSP Grantees</b>		
New York	<ul style="list-style-type: none"> <li>Up to 3 consumers as paid staff</li> <li>Assisted with French translation of patient diary</li> </ul>	<p><b>Facilitators</b></p> <ul style="list-style-type: none"> <li>Consumer staff very helpful as mentors for support groups</li> </ul> <p><b>Barriers</b></p> <ul style="list-style-type: none"> <li>Very difficult to engage parent partners due to their childcare needs</li> </ul>

*“Working with the staff and the healthcare providers at UC Health and the Ohio Valley Sickle Cell Network has been a truly wonderful and insightful experience. As a result of working together over two years I have gotten to know and grown closer with them, which has had a positive impact on my care at UC Health. Having healthcare providers who really know you over the course of years, makes being a patient a less frustrating and complicated experience. Moreover, knowing about the hard work and the challenges that they face to provide services to their patients has allowed me to have a greater appreciation of the care I receive. Lastly, working with these professionals has expanded my knowledge of sickle cell anemia allowing me to become a more informed patient, which has enabled me to better enlighten people in the community in my role as volunteer and chairman of our community outreach group S.CELL.”*

*– OH SCDTDP patient*

## INFLUENCE OF CONTEXT ON QUALITY IMPROVEMENT

There is significant research on how contextual factors influence the level of success in quality improvement initiatives.<sup>27,71,72,73,74,75</sup> Context can be defined as “anything not directly part of the technical quality improvement process that includes the quality improvement methods themselves and the clinical interventions,” including factors relating to the organization, the individual, the individual’s role in his or her organization, and the environment in which the organization exists. A deeper understanding of context provides insight into the story behind the outcomes for the grantees. This understanding can inform and improve the development and implementation of future sickle cell disease-related initiatives. In order to examine the role that contextual factors played in the SCDTDP and SCDNBSP grantees’ success, NICHQ developed a framework that synthesizes the current literature around context in quality improvement, particularly from the Model for Understanding Success in Quality Improvement (MUSIQ), and NICHQ’s experience in conducting and evaluating quality improvement initiatives. As described in Section 1, this framework was adapted specifically for the SCDTDP (see Appendix 4).

The framework was shared with SCDTDP grantees at Learning Session 6 of the Hemoglobinopathy Learning Collaborative, in May 2014. Each grantee was asked to rate every secondary level of the framework in terms of how that level influenced its work overall on a scale of 1: significant barrier to 5: significant facilitator, and to provide detailed comments about key factors that were facilitators or barriers to their work at each level. The NICHQ evaluation team revised the framework based on this feedback. The resulting framework provides an overview of the key contextual factors at various levels influencing the degree of success of the grantees in their improvement work.

**FIGURE 21:**

*Influence of SCDTDP contextual factors*

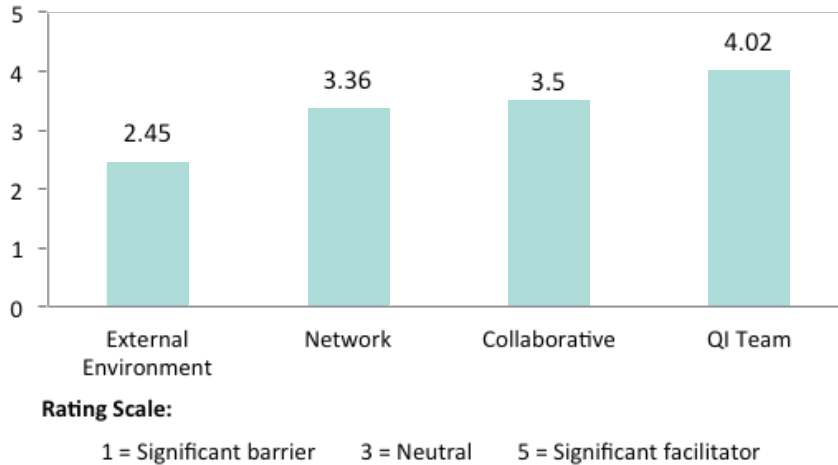


Figure 21. Average rating of contextual factors levels across all grantee networks. In general, network, collaborative, and team factors were considered facilitators while external environmental factors were considered barriers to achieving results.

The framework has four primary levels: 1) external environment (e.g., demographics, state-level funding); 2) network (i.e., multiple practices and sites collaborating together); 3) learning collaborative (e.g., structure, faculty); and 4) improvement team (e.g., composition, leadership). Each primary level has corresponding secondary levels and more detailed components. On average, the external environment level was found to be more of a barrier than any of the other four contextual levels, whereas the improvement team was more of a facilitator than any of the other levels (Figure 21).

## ASSESSING HEALTHCARE USE AND HEALTH-RELATED QUALITY OF LIFE

The previous section presented rich quantitative and qualitative data demonstrating the breadth and depth of quality improvement undertaken by grantees, as well as their work partnering with patients and families, educating diverse stakeholders, engaging communities and developing tools and resources. The section that follows presents the quantitative data collected annually through survey instruments designed to assess self-reported patient health status, health care utilization and health-related quality of life. These two data streams – the first collected primarily by providers and health care systems and the second collected through patient self-report – complement one another by capturing data to illustrate the work done to improve care for individuals with sickle cell disease from both providers and patients.

### METHODS

#### Survey Participants

We fielded a client survey instrument, the Individual Utilization Questionnaire (Appendix 6) with patients with sickle cell disease enrolled in the SCDTDP from June 17, 2008 to June 17, 2014 to better understand sickle cell disease-related health care utilization and morbidity. Through the surveys we also sought to examine health-related quality of life for these patients through two questionnaires: the Pediatric Quality of Life Inventory™ (PedsQL™)<sup>77</sup> and the SF-8™ Health Survey (SF-8),<sup>78</sup> included as Appendices 7 and 8 to this report. A convenience sample (i.e., a sample of the most available subjects in the population used to obtain results) of patients utilizing care at nine participating centers nationwide was recruited for the survey. The survey mode was face-to-face interviews with patients by trained staff at each site. Comprehensive baseline data were collected at enrollment and follow-up data collection was attempted for each enrolled participant once per 12 months thereafter for a possible total of two follow-up periods.

#### Survey Content

The Individual Utilization Questionnaire assessed the following: demographics, income and educational status, type of disease, age at diagnosis, utilization of services, hospitalization, complications, and treatment status including antibiotics, hydroxyurea, transfusion, and counseling. These were collected based on patient self-report or parent report (in some cases data were confirmed by medical record). Immunization history was collected based on medical record. Analysis for change over time (longitudinal analysis) is presented based on matched baseline and follow-up data for 1,642 patients.

The two instruments used to assess quality of life for patients living with sickle cell disease, the SF-8 and PedsQL™, are 8-item and 15-item surveys, respectively.<sup>77,79</sup> These scales have been used in large population studies.<sup>80, 81, 82, 83, 84</sup> Both the PedsQL™ and SF-8 provide a health profile that encompasses physical and emotional health. Specifically, the SF-8 provides summary scores for the following domains of health in adults: physical functioning, role limitations due to physical health, bodily pain, general health perceptions, vitality, social functioning, and role limitations due to emotional problems and mental health. The PedsQL™ measures health-related quality of life specifically in children and adolescents, through brief, practical, generic core scales such as performance in general physical health, emotional health, social skills, and in academics. It is administered to both parents and pediatric patients.

## Statistical Methods and Analyses

Demographic variables such as gender, race, sex, ethnicity, and sickle cell disease type were assessed in order to understand the sample population. Analyses were conducted to assess changes in health care utilization measures over time. Trend analysis was also performed for reported outcomes including hospitalizations, emergency department visits, hydroxyurea use, and hydroxyurea counseling.

The responses from the SF-8 and PedsQL™ surveys were scored and analyzed as overall measures of level of functioning. PedsQL™ scores for this study were compared to those presented from other studies.<sup>79,81,85,86,87</sup> Comparison scores for SF-8 were not presented because they were not available for patients with sickle cell disease.

The last set of analyses compared data from the prior National Coordinating Center and the current National Coordinating Center to explore whether outcomes differed significantly between the time periods covered by each. A detailed description of the statistical methods employed can be found in Appendix 3 and all tables and figures can be found in Appendix 9.

## RESULTS AND IMPACT

Our analysis of the client survey data highlights the feasibility of conducting multi-state data collection in patients with sickle cell disease, although completion of follow-up surveys was variable between sites (Appendix 9, Table 1).

### Demographics

Surveys conducted over the six-year period from June 2008 to June 2014, a total of 1,743, were analyzed including those from baseline (first encounter n=1040) and the three follow-up periods (each about 12 months apart) with 602, 80, and 21 surveys, respectively. Of the 1,040 unique patients participating during the project period, 58 percent completed a first follow up. Appendix 9, Table 1 describes the distribution of the sample across the nine sites.

The patient population was predominantly female (60.0 percent) and the average age was 24 years (Appendix 9, Table 2). There was significant difference in age across sites. Medicaid (65.9 percent), Medicare (19.2 percent), and private insurance (19.9 percent) accounted for major types of insurance for the patients completing the surveys. The majority (94 percent) of the patients were African American while 2.5 percent were of Hispanic ethnicity. Sickle cell disease is estimated to occur in 1 of 500 African-American births, with higher rates among immigrants from Africa and the Caribbean. Our survey population is similar to what has been reported previously.<sup>3,4</sup> However, as the population of individuals of Hispanic origins has been growing steadily, it is important to pay close attention to this emergent population.<sup>88</sup>

Almost three-quarter (72 percent) of the patients reported sickle cell anemia, the most severe genotype, while 15.5 percent reported sickle hemoglobin C disease. Genotype was also checked via the medical record and there was a statistically significant difference ( $p < 0.001$ ) in self-reported disease type and that documented in the medical record (Appendix 9, Table 3). This discrepancy in patients' knowledge about their disease types and those reported in the medical records could be due to issues in communication to patients and their families.

The newborn screening program identified slightly more than half (54.8 percent) of the participants and 92.9 percent of participants born after 2006, when all 50 states and Washington D.C. adopted universal screening programs. The mean age of diagnosis for those not diagnosed by the newborn screening program was 5.5 (range: 0-52; SD of 7.8). It is possible that those not diagnosed during the newborn period were immigrants or that these patients were born before newborn screening was implemented in their state.<sup>89</sup> Because early diagnosis and routine health care is key in the management of sickle cell disease, our finding that the mean age at first diagnosis was 5.5 highlights an opportunity to enhance screening, follow up and communication of results.

## Healthcare Utilization

In examining healthcare utilization, there was no significant difference in patient visits to their physicians between baseline and follow up for both sickle cell disease and non-sickle cell disease-related physician visits (Appendix 9, Table 4). There was also no change seen between baseline and follow up for emergency department visits (baseline: 4 vs follow up: 3.5;  $p=0.08$  – Appendix 9, Table 5). The percentage of patients admitted to the hospital reduced significantly at follow up (63.8 percent baseline vs. 56.6 percent follow up;  $p=0.004$ ), but there was no difference in the average number of hospital stays between baseline and follow up (2.3 vs. 2.4;  $p=0.37$  – Appendix 9, Table 5). When attempts were made to adjust for type of sickle cell disease, there was no significant decrease in hospitalizations. However, there were a limited number of observations available to allow for adjusted analysis. Further observations would be needed to interpret these data sufficiently.

## Sickle Cell-Specific Medications and Treatment

There was a statistically significant decline in regularly scheduled blood transfusions between baseline and follow up (40 percent vs. 26.7 percent;  $p<0.0001$  – Appendix 9, Table 6). This could be due to changing practice patterns.

In terms of prophylactic antibiotic use in children under the age of 5 to prevent infections, we found no difference in antibiotic use between baseline and follow up (baseline 96.7 percent vs. follow up 96 percent – Appendix 9, Table 7).

There was no difference in hydroxyurea use (38.3 percent baseline vs. 41.2 percent follow up;  $p=0.25$  – Appendix 9, Table 5) or counseling for hydroxyurea use (41.1 percent baseline vs. 42.6 percent follow up;  $p=0.66$  – Appendix 9, Table 5).

## Health Outcomes and Complications

When we examined known complications from sickle cell disease in this survey population (Appendix 9, Figure 1), the prevalence of most complications was significantly less at follow up compared to baseline measures (Appendix 9, Table 8).

Complications due to sickle cell disease are widely reported in several studies.<sup>90</sup> While we found a significant decline in most of the common complications, it is difficult to interpret the significant drop in follow up because of the nature of the format of the original question's wording, where the survey item's stem is worded differently based on the type of interview. At baseline, the question is "(Have you/has the client) ever had the following sickle cell complications?" and at follow up the question is "In the past 12 months, (have you/has the client) had the following sickle cell complications?" This can be confusing because at baseline the question is focused on assessing prevalence, while at follow up, the question is focused on incidence (i.e., new occurrences).

## Sickle Cell Specific Screening/Counseling

Three-quarters (75.4 percent) of patients received counseling for sickle cell disease-related complications at baseline, while significantly fewer (61.8 percent) received counseling for the same at follow up ( $p<0.0001$ ). Similarly, about 77.1 percent of clients receive counseling on sickle cell disease inheritance at baseline while significantly fewer (50.1 percent) receive that counseling at follow up ( $p<0.0001$ ) (Appendix 9, Table 9). Similar to the question on complications, it is difficult to interpret the significant drop in follow up because of the nature of the format of the original question's wording, where the survey item's stem is worded differently based on the type of interview. Grantees did anecdotally report that counseling was not done consistently at follow up because clients gain more experience in dealing with their sickle cell disease-related complications and do not see a benefit from additional counseling. Additionally, comprehensive counseling was done at baseline so there is little need or time for it during follow up when attention was given to other issues, such as chronic conditions for older patients.

## Trend Analysis

Appendix 9, Figures 2-4 reflect the trend during this program period for three main outcomes of interest: emergency department visits, frequency of hospitalizations, and hydroxyurea use. After statistical adjustments, emergency department visits have decreased slightly over time, but this decrease is not statistically significant (Appendix 9, Figure 2). The frequency of hospitalizations, categorized from one to five, has decreased over this study period and is statistically different (Appendix 9, Figure 3). Finally, our data show a significant slight upward trend in hydroxyurea use over time, for both weighted and un-weighted analyses (weighted  $p=0.0079$ , un-weighted  $p=0.0157$ ) (Appendix 9, Figure 4).

## Quality of Life Measures

Over the six years, patients were given two sets of surveys (PedsQL™ and SF-8 Health Survey) to assess their quality of life living with sickle cell disease. The surveys explored the physical, functional, and psychological issues in patients and their families with sickle cell disease. The data (described in detail below) were not significantly different between baseline and follow up suggesting that quality of life outcomes presumably require longer time intervals to see significant change. The mean functioning score for our sample was higher than those published in the literature for patients with sickle cell disease but lower than those reported for the general population.<sup>26, 80, 86, 91, 92, 93</sup>

### PedsQL™

The PedsQL™ survey has two respondents: parent and child (child data not presented due to limited sample size). There were 330 parent participants at baseline and 204 at a 12-month follow up visit in 8 network sites (Appendix 9, Table 11). Data are presented as overall, baseline, and follow up (Appendix 9, Table 12). The demographic distribution of the participants as well as the genotype diagnosis is comparable to the overall convenience sample described earlier. The composition of this population was: 80.3 percent African Americans, 1.5 percent White, 1.5 percent Native Hawaiian/PI, 0.3 percent Asian, 1.2 percent Native American. Hispanic ethnicity accounted for 2.6 percent of the sample. Females represented 55 percent of this sample. The average age for the patients was 9 years. The responses to the quality of life questions that were covered in the four domains were not statistically different between baseline (N=314) and follow up (N=130). The overall mean scores for each domain are 74.4 for physical functioning, 73.6 for emotional functioning, 79.6 for social functioning, and 66.4 for school functioning (Appendix 9, Table 12). These findings were consistent across the eight sites.

### SF-8 Health Survey

There were 639 patients that completed the SF-8 survey at baseline and 288 at follow up (Appendix 9, Table 14). The racial/ethnic distribution for this group of respondents was: 74 percent Black/African American, 3.1 percent American Indian, and 1.3 percent White. The average age of the respondents was 32.3 years (SD =12). The responses to the quality of life questions that were covered in the five domains (Appendix 9, Table 15) were not statistically different between baseline and follow up, similar to what we observed in the PedsQL™ survey. These findings were consistent across the nine sites.



## Cross Sectional Analysis: Comparison of Outcomes between Prior and Current National Coordinating Center

We had an opportunity to compare data from the grantees participating in the programs run by the prior and current National Coordinating Centers. 1,109 participants completed surveys under the prior National Coordinating Center (June 2008 to September 2010) and 1,221 participants completed surveys under the current National Coordinating Center (September 2010 to June 2014).

The demographic distributions are similar except for distribution of gender between patients during the prior and current National Coordinating Center (Appendix 9, Table 16). The current National Coordinating Center had a significantly higher proportion of female patients ( $p < 0.0001$ ). While hospitalizations remained stable over time, hydroxyurea use and counseling about hydroxyurea increased significantly for participants during the current National Coordinating Center. Patients participating in the current program were 1.5 times more likely to use hydroxyurea than patients during the prior program (OR=1.5, 95 percent CI 1.3-1.8,  $p < 0.0001$ ). The odds of receiving counseling for hydroxyurea among those not already using hydroxyurea were 1.6 times higher than patients involved during the prior National Coordinating Center. (OR=1.6, 95 percent CI 1.3-2.0,  $p < 0.0001$ ).

There was a significantly greater proportion of patients receiving hydroxyurea counseling as well as hydroxyurea during the period of the current National Coordinating Center. We cannot determine whether this is due to selection of sites between the two National Coordinating Centers, changes made specifically by grantee networks as a result of their participation, or changes that would have occurred regardless (and may be similar in non-program sites), as hydroxyurea use has generally increased with better understanding of the treatment and its side effects.<sup>24</sup>

## CONSIDERATIONS FOR UNDERSTANDING DATA ON HEALTH SERVICES AND HYDROXYUREA USE

The previous sections describe the results from two streams of data that were collected for this project: (1) quality improvement data derived from the medical record, collected on a monthly or quarterly basis, which served to monitor and drive improvements in care processes and outcomes and to inform collaborative activities, and (2) client survey data collected annually throughout the project period to assess self-reported patient health status, health care utilization and health-related quality of life.

Each of the data sources that were used to assess program impact has limitations that need be considered in interpreting the findings.

Delays in the Office of Management and Budget providing approval for the quality improvement data collection resulted in most of these measures only being collected for a short period of time at the end of the project period. Grantees began making changes in their systems long before data were collected. Consequently, grantees may have already realized improvement in some domains of care not reflected in these data. In addition, grantees did not have the benefit of being able to learn from their data or the data of their colleagues and use this learning to modify their approaches.

In addition, the quality improvement data were reported for a convenience sample of patients seen at grantee sites each month. These patients do not represent a random sample of all patients cared for at these sites (or even of those having visits), and those who visit may be sicker or more conscientious than those not visiting. Another limitation of the QI data is that the measures of hospitalizations and emergency department visits do not include data about care obtained at sites outside of the networks.

The client survey data are also a convenience sample of a variable number of patients at each site. In addition, sites reported follow up data on a little more than half of those initially surveyed, providing much opportunity for bias based on who did or did not follow up. Although some types of information—such as quality of life—can only be obtained by survey, patients may not be accurate reporters of other types of information, such as use of medication or health care visits.

Although the quality improvement data primarily focused on processes of care and the self-reported client survey data primarily assessed health outcomes, a few measures were reported through both data streams: hydroxyurea use, hospitalizations, emergency department visits and physician visits. The nuances between the two data sets for these measures, as well as our interpretation of the results, are described in detail below.

### Hydroxyurea

Since 2008, when the National Institutes of Health Consensus Conference on Hydroxyurea highlighted that hydroxyurea was underused, concerted education efforts focused on the benefits and risks of this treatment have increased provider and patient awareness of hydroxyurea.<sup>62</sup> Analysis of client survey data from 2008 (from current grantees also participating in the prior SCDTDP) to 2014 (the end of the current program), revealed a statistically significant increase in hydroxyurea use. This is consistent with the broad trend noted above, although may be due to focused efforts from project teams not captured in our data. Quality improvement data related to hydroxyurea use collected as part of the Hemoglobinopathy Learning Collaborative from 2013 to 2014 showed that 50 percent of eligible patients were on hydroxyurea. Although this percentage did not increase during the brief period that quality improvement

data were reported to the National Coordinating Center, the percent of patients on hydroxyurea in the SCDTDP and SCDNBSP grantees is higher than published reports of hydroxyurea use.<sup>12, 95, 96</sup> Again, this could be the result of enhanced efforts to increase the use of hydroxyurea at participating sites. Finally we note that one grantee, Massachusetts SCDNSBP, that assiduously applied the quality improvement approach employed by the National Coordinating Center and obtained data over a more extended period of time demonstrated improvement in the percentage of patients taking hydroxyurea to a substantially higher level –75 percent – suggesting that further improvement is possible and that the approach can indeed work (Figure 16).

### **Emergency Department Visits**

Neither the quality improvement nor client survey data showed any change in the number of sickle cell disease-related emergency department visits over the course of the project. Although we had hoped increased use of hydroxyurea or better self-management strategies may have decreased the frequency of complications resulting in emergency department visits, the improved access to care and education about appropriate signs and symptoms requiring emergency treatment may have counterbalanced any such positive effects.

### **Hospitalizations**

The quality improvement data for the percentage of patients with at least one hospitalization from June 2013 through May 2014 show a slight decrease over several months, but this decline was not sustained over time. Similarly, the raw data based on the client survey data showed a reduction in the percentage of patients admitted to the hospital between baseline and follow up; however, when the precise type of sickle cell disease was taken into account (as some variations result in more severe disease), this difference was not statistically significant. There were, however, promising signals detected through both approaches.

Based on the quality improvement data (derived from chart review), approximately 10 percent of patients experienced at least one hospitalization. In the client survey data, the percentage of patients admitted to the hospital was much higher than that reported in the quality improvement data (63.8 percent at baseline and 56.6 percent at follow up). The discrepancies between these data may be due to patients seeking care at sites outside of grantees and thus not being represented in the quality improvement data derived from chart review at a single institution or network. In addition, patients may include hospitalizations for time periods longer than a year when asked by a survey, and the mechanism for identifying patients for the client survey may have resulted in oversampling patients at the time of a hospitalization.

### **Patient Visits to Primary Care Providers and Specialists**

Quality improvement data on the percentage of patients who had an evaluation with a hematologist and the percent of patients who had a documented visit with their primary care provider improved over the course of the project for the four grantees that consistently reported on those measures. The client survey data from all SCDTDP grantees revealed that average number of visits to a primary care provider and sickle cell specialist remained stable between the baseline and follow-up periods.

In addition to reporting data from a different number of grantees, the quality improvement and client survey data are reporting on slightly different measures. The quality improvement data is reporting on the percentage of patients that have had at least one visit with a provider, while the client survey data is reporting on the number of visits to a provider. Both data reveal that patients had access to primary care providers and sickle cell specialists during the project period. The quality improvement data suggest that grantees that focused on these processes were able to improve the likelihood of patients having at least one visit thus advancing the goal of patients have continued access to primary care providers and specialists.

## LESSONS LEARNED AND RECOMMENDATIONS

### LESSONS LEARNED

Sickle cell disease is a chronic condition disproportionately affecting our country's most vulnerable populations, many of whom experience fragmented, poor quality and often less than humane care.<sup>38,97</sup> Improving the quality of care and overall health of individuals living with sickle cell disease is a critical challenge, one that requires a multifaceted approach. Multiple stakeholders including patients, family members, primary care providers, specialists, community based organizations, public health and payers need to work collaboratively to ensure individuals with sickle cell disease have access to a holistic system of care that ultimately leads to optimal health.

Ten years ago, the Sickle Cell Treatment Act provided funding for projects to demonstrate ways to improve care and outcomes for individuals affected with sickle cell disease.<sup>98</sup> For the past four years, NICHQ and its partners supported these grantees using collaborative learning and quality improvement. This approach entailed regularly collecting data and sharing results and best practices among the grantees, providing great opportunity for learning. The current Sickle Cell Disease Treatment Demonstration Program has made great strides in improving the quality of care for individuals with sickle cell disease. Grantees were able to apply improvement science methods to make improvements in several processes of care that positively affect patients. These improvements included more timely and compassionate care in emergency departments, increased access to providers, and more reliable provision of recommended screenings and therapies. The encouraging results and work described in this report have provided a number of important lessons:

1. Targeted strategies implemented using a disciplined change approach can lead to significant improvements in the quality and timeliness of treatment in the emergency department and enhance patient experience of care.
2. Use of patient navigators, community health workers, community-based organizations and patient self-management tools can improve access, coordination and integration of services for patients with sickle cell disease.
3. An early and comprehensive approach to transition, combined with self-management support can help mitigate the many challenges that individuals with sickle cell disease face during this vulnerable time.
4. Multilevel interventions targeted at the patient, family, provider and system can increase hydroxyurea use.
5. Opportunity still exists to improve follow up care after screening to ensure patients are enrolled in comprehensive care. Further work is needed to identify the appropriate processes for screening immigrant populations for sickle cell disease.
6. A shared and coordinated measurement strategy across grantee networks can enhance the program's ability to measure improvements in key process and outcomes related to sickle cell care. Coupling the measurement with a systematic approach to improvement results in better care and will ultimately lead to better outcomes.

## RECOMMENDATIONS

The accomplishments of the grantees over the past four years demonstrate the impact that can be realized when patients and families, providers, community based organizations, public health and government agencies work collaboratively to improve care for individuals with sickle cell disease. At the same time, the challenges the sites and we encountered, the limits in what they and we accomplished inform our recommendations as well. NICHQ shared draft recommendations with grantees, faculty, and Oversight Steering Committee members at several stages, and NICHQ convened multiple brainstorming sessions to identify and interpret key findings. Through feedback and discussion, the diverse perspectives of all stakeholders were incorporated. The data and feedback were synthesized using the content expertise of the project team, and final recommendations were finalized by NICHQ. These recommendations address several different levels of action: (1) Recommendations for clinical delivery and public health programs, (2) Recommendations for the design or re-design of the Sickle Cell Disease Treatment Demonstration Program and (3) Recommendations for broad health policy.

The system of care for individuals with sickle cell disease should include the main tenets of the patient-centered medical home, and the overall goal should be to move beyond simply demonstrating how to improve care for these individuals to spreading these improvements so that all patients with sickle cell disease have access to a system of high quality care. All of the recommendations included in this report are directed towards achieving the aim of the Sickle Cell Treatment Act, which is to improve the health care and outcomes for individuals with sickle cell disease.

### I. Recommendations for Clinical Delivery and Public Health Programs:

- a. Address deficiencies in emergency department care of individuals with sickle cell disease experiencing acute pain crises by establishing pain protocols, providing and making widely available pain management plans and using more easily administered medications.
- b. Continue to increase access to medical homes and enhance care management and care coordination through the use of care management plans jointly developed by primary care providers, specialists, hospitalists and other inpatient providers with patients and families.
  - i. Expand the evidence base related to the use of care plans and other care coordination tools in sickle cell disease.
- c. Implement systems (e.g., electronic health record templates, order sets, tracking and feedback mechanisms) to increase rates of appropriate screening and preventative interventions (e.g., penicillin prophylaxis, immunizations, hydroxyurea, transcranial Doppler screening).
- d. Ensure education regarding use of hydroxyurea extends beyond a discussion of benefits and risks to include discussion of patient preferences and strategies for self-management support.
- e. Ensure that health care systems address psychosocial needs of individuals with sickle cell disease and their families as well as medical needs.
- f. Ensure all facilities providing care for individuals with sickle cell disease incorporate the six core elements of transition where appropriate, including 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.
- g. Assess current practice patterns for screening of immigrants (including African, Caribbean, Hispanic and Middle Eastern immigrants) for sickle cell disease. Develop and/or refine screening processes and link identified individuals to systems of care based on this assessment.

- h. Involve patients and families in the design and implementation of quality improvement activities.
- i. Involve community-based organizations as partners in programs to improve care for individuals with sickle cell disease across the lifespan.
- j. Implement data systems that enable management of the entire sickle cell disease 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., hydroxyurea), emergency department visits, hospitalizations, and readmissions.
- k. Use systematic approaches to quality improvement, based on data, family engagement, and evidence.

## 2. Recommendations for the Design or Re-design of the SCDTDP:

- a. We endorse the focus of the new SCDTDP on increasing access to care, increasing the number of providers capable of caring for individuals with sickle cell disease and increasing the use of hydroxyurea, as well as adopting a regional model to spread improvements in care across broader sections of the country.
- b. Resources of the SCDTDP should be aligned with prevalence of sickle cell disease, perhaps initially allocating resources to those regions with higher numbers of affected individuals with a future plan to expand resources to ensure all patients irrespective of geographic location have access to high quality care.
- c. Until all patients with sickle cell disease have access to high quality care, consider implementation of telehealth strategies to ensure patients have some access to services even if they are not close to a sickle cell program or center.
- d. Involve patients and families in program development and program activities to ensure that efforts are responsive to their ongoing needs.
- e. Financial and technical support for data collection should be commensurate with programmatic needs; the current resources are grossly insufficient to collect and report on the necessary data elements.
- f. The Health Resources and Services Administration should align funding cycles of the National Coordinating Center and program grantees to ensure similar start and end dates.
- g. The Health Resources and Services Administration should require the National Coordinating Center and program grantees to adopt a shared measurement strategy and data collection system.
- h. Improvement science should remain an integral component of the SCDTDP.
- i. Interagency coordination and cooperation could amplify the impact and optimize the resources of the SCDTDP. This can occur across the bureaus of the Health Resources and Services Administration, e.g., through engagement with the Bureau of Primary Health Care, as well as across other agencies within the Department of Health and Human Services and beyond. These other agencies include the Centers for Disease Control and Prevention, the Centers for Medicare and Medicaid Services (including its Center for Medicare and Medicaid Innovation), the Agency for Healthcare Research and Quality, the National Institutes of Health, the Office of Minority Health and others.
- j. The work of the SCDTDP and the SCDNBSP should be aligned. Collaboration between grantees and the coordinating centers will maximize resources and impact while limiting duplication.

### 3. Recommendations for Health Policy:

The health care needs of this population should be addressed through broadly implemented health policies rather than relatively small demonstration programs. Specific policy options might include:

- a. New payment models that ensure that all patients with sickle cell disease have consistent insurance access to high quality care that is linked to a quality performance reporting and improvement system (e.g., categorical eligibility for Medicare for patients with sickle cell disease, analogous to individuals with end-stage renal disease, regardless of age).
- b. Adjusting Medicaid payment policies and enhancing reimbursement rates to include care coordination services for this population, as was recently implemented for Medicare.
- c. The Center for Medicare and Medicaid Services should develop risk-based capitation strategies for sickle cell disease.
- d. Consider specific reporting on readmissions for sickle cell disease in hospitals; this might be paired with financial incentives with appropriate adjustment for severity of illness and other indicators of risk.
- e. Adopt recently developed performance measures for sickle cell disease into insurance programs (Medicaid, Children's Health Insurance Program, Medicare) across the lifespan. Incorporate these measures, or a subset of them, in the Bureau of Primary Health Care quality performance measures.
- f. Specific workforce training programs for health care professionals interested in caring for individuals with sickle cell disease. Provide enhanced compensation and potential loan forgiveness programs for hematologist/oncologists committing to at least a minimum number of patients with sickle cell disease or proportion of their practice devoted to patients with sickle cell disease.
- g. Incorporate sickle cell disease-specific requirements in federal regulations for meaningful use.
- h. More broadly, assure that all federally supported health care programs (e.g., federally qualified health centers, Department of Defense and Department of Veterans Affairs health care programs) apply the clinical recommendations noted above.

The current Sickle Cell Disease Treatment Demonstration Program has demonstrated that better care for individuals with sickle cell disease is possible. This report has synthesized what can and should be done to improve care and provided recommendations for how these improvements can be implemented. The recommendations regarding modifications to the Sickle Cell Disease Treatment Demonstration Program provide an opportunity for how this program can enable even greater learning and have greater impact on the populations directly touched by grantee programs. Yet what are most needed are mechanisms to move these lessons into widespread practice and to address barriers (such as an insufficient provider workforce) beyond the scope of the currently designed program.

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APPENDIX I

**SICKLE CELL DISEASE TREATMENT  
DEMONSTRATION PROGRAM  
MODEL PROTOCOL**  
AND COMPENDIUM OF RESOURCES:

## INTRODUCTION

Between 2010 and 2014 Nine Sickle Cell Disease Treatment Demonstration Program (SCDTDP) funded grantee networks from across the United States applied the principles of collaborative learning and improvement science to improve processes and systems of care for individuals living with sickle cell disease. The National Institute for Children's Health Quality (NICHQ) served as the National Coordinating Center during this period.

This model protocol includes recommendations regarding the highest-leverage changes that led to process improvements across five dimensions of sickle cell care listed below in the Hemoglobinopathy Learning Collaborative, sponsored under the auspices of Health Resources and Services Administration (HRSA) and funded by the SCDTDP:

1. Acute care
2. Medical home/care coordination
3. Screening and follow up
4. Transition of care
5. Hydroxyurea

The purpose of this model protocol is to provide clinicians, nurses, allied health professionals, community-based organizations and public health agencies with recommendations and strategies to improve care provided to individuals with sickle cell disease and trait. The National Coordinating Center strongly encourages organizations to develop an integrated advisory committee interested in sickle cell disease care comprised of multiple stakeholders including patients, parents, family members, community health workers or patient navigators, physicians, nurses and allied health professionals. These advisory committees should review these recommendations and consider testing and adapting some of these changes in their respective settings.

The majority of the recommendations result from a synthesis of changes implemented across the grantee networks that led to process improvements. NICHQ also reviewed and included some recommendations from existing published clinical practice guidelines and consensus statements related to the care of individuals with sickle cell disease. Lastly, the model protocol includes guidance from expert panels consisting of health care professionals with expertise in hematology, pediatrics, newborn screening, genetics and public and community health convened by NICHQ for the SCDTDP and the Sickle Cell Disease Newborn Screening Program (SCDNBSP). A systematic assessment of the quality of evidence associated

with each recommendation was beyond the scope of the project, and some recommendations may highlight areas where future research is warranted given a limited existing evidence base. The model protocol was reviewed by representatives from all of the SCDTDP grantee networks, including patients and family members of patients, as well as the SCDTDP Oversight Steering Committee and HRSA program staff.

## ORGANIZATION OF MODEL PROTOCOL AND COMPENDIUM OF RESOURCES:

The model protocol includes a section for each of the dimensions of sickle cell care in which grantee networks worked: acute care, care coordination and self-management, screening and follow up, transition and hydroxyurea. This [model protocol] is not a comprehensive listing of changes for every dimension of sickle cell care but rather includes recommendations on the topics where SCDTDP grantee networks focused their efforts during the funding period. Each section includes an overview of the specific topic, including rationale for why it is important to improve this dimension of sickle cell care, and a discussion of the recommendations for high-leverage changes. The resources listed in each section of the model protocol were used by teams as they implemented the high-level changes in their organizations. The companion compendium of resources includes educational materials for patients and providers such as clinical algorithms, standardized order sets, and patient tracking tools.

## ACUTE CARE

Acute vaso-occlusive episodes, often referred to as pain crises, are unpredictable bouts of pain that are the most common reason for emergency department visits and hospitalizations for patients with sickle cell disease.<sup>1,2,3,4</sup> Timely and appropriate use of oral or parenteral analgesia (i.e., pain medication) can result in pain relief, reduce hospitalizations and reduce the development of chronic pain syndromes.<sup>5</sup> Both pediatric and adult patients with sickle cell disease experience prolonged periods of waiting for pain medications in the emergency department despite the existence of detailed guidelines<sup>6,7</sup> and quality indicators<sup>8</sup> related to the management of pain crises.<sup>9,10</sup> Emergency department visits and hospitalizations account for a significant proportion of health care expenditures in this population.<sup>11</sup>

An important component in improving key processes in the management of pain crises in the emergency department is

forming a multidisciplinary group comprised of patients and family members, providers from the emergency and hematology departments, and other physicians, nurses, nurse practitioners, psychologists, pharmacists and allied health professionals such as community health workers. Key responsibilities for this team include identifying a physician and/or nurse “champion” from the

emergency department, inviting individuals with sickle cell disease to review performance data and provide ideas to inform the initiative, openly sharing data with affected individuals and emergency department staff, and offering trainings and educational materials to nursing and physician staff.

## RECOMMENDATIONS:

1. Rapidly triage patients and assess recent use of pain medications and quality and location of patient’s pain. Use age-appropriate pain assessment tool to assess intensity of pain.
2. Analgesia should be rapidly started within 30 minutes of triage or within 60 minutes of registration.
3. Use standard order sets for management of sickle cell pain in acute care settings such as the emergency department and, when appropriate, use individual pain treatment plans to facilitate timely, effective and safe management of pain crises.
4. Reassess in regular intervals (e.g. 30 minutes) after each dose of pain medication for pain relief and side effects.
5. Regularly assess patient and family satisfaction with and experience of care in acute care setting.
6. Regularly track performance on timeliness of assessment and reassessment of pain and administration of pain medications to assess impact of process improvements.
7. Consider initiating patient-controlled analgesia for patients who will be admitted to the hospital for pain management.
8. Consider use of intranasal fentanyl as a short-term intervention to relieve pain when intravenous access is difficult or until intravenous access is obtained.

**TABLE 1:** High-leverage changes and resources tested by grantee networks in acute care

Change Idea	What is it? (Definition) Why do we use it? (Rationale)	Resources
Pain assessment charts	Since pain is often subjective and personal, pain assessment charts help patients describe the amount of pain an individual is feeling. Numerical and picture-based charts allow patients to communicate their pain more clearly so that interventions can be planned accurately.	<a href="#">Illinois SCDTDP Pain Chart</a> <a href="#">Wong-Baker FACES Pain Rating Scale®</a>
Standard order sets	Standard order sets are a group of medical orders used to standardize diagnosis and treatment for specific medical conditions such as sickle cell pain based on clinical practice guidelines. These order sets communicate best practices, reduce variation and potential for medical errors, and enhance workflow. In this context, the order set standardizes the timeframes for triage, medication administration, and reassessment of pain with the goal of expediting patient care and decreasing delays in critical interventions such as administration of pain medication. Standard order sets can be paper-based or embedded in an electronic health record system.	<a href="#">California SCDTDP Sickle Cell Initial Order Set</a> <a href="#">Massachusetts SCDNSBP Pediatric ED VOE Protocol</a> <a href="#">New Jersey SCDTDP ED Algorithm</a> <a href="#">Tennessee SCDNBSP Checklists for Pain, Acute Chest, Stroke and Iron Overload</a>

Change Idea	What is it? (Definition) Why do we use it? (Rationale)	Resources
Pain action plans	Individual pain action plans list pain medication and doses that have been previously effective for that individual. Tailoring pain treatment to the individual facilitates faster and more effective pain management.  Care plans should be developed and finalized with patients and their families based on their desired level of engagement.	<a href="#">California SCDTDP (English and Spanish) Pain Action Plan</a> <a href="#">Massachusetts SCDNBSP Adult ED Individualized Pain Plan</a> <a href="#">Pennsylvania SCDNBSP Pain Action Plan</a>
Patient satisfaction surveys	Surveys allow individuals to let clinic staff know which parts of care worked well and which were less than ideal. Obtaining feedback from patients and families allows improvement teams to determine what areas need to be addressed more urgently than others.	<a href="#">Massachusetts SCDNBSP Pediatric ED Satisfaction Survey</a> <a href="#">Massachusetts SCDNBSP Adult ED Satisfaction Survey</a>
Patient-controlled analgesia pumps	A computerized pump which contains a syringe of pain medication prescribed by a physician is connected directly to a patient's intravenous line.  Patient-controlled analgesia pumps allow patients to control the timing of intravenous administration of their own pain medication, resulting in timely pain relief.	<a href="#">Massachusetts SCDNBSP PCA Handout</a>
Intranasal fentanyl	Opioid analgesic administered intranasally (a squirt into the nose) to allow for rapid administration of first dose of pain medication while awaiting IV access or if IV access is difficult. This medication comes in a liquid preparation and is not available over the counter. Further studies are being conducted to assess the impact of this medication on subsequent doses of parenteral analgesia.	<a href="#">Massachusetts SCDNBSP Intranasal Fentanyl handout</a> <a href="#">California SCDTDP ED Protocol for IN Fentanyl</a>

## MEDICAL HOME/CARE COORDINATION

Care for persons with sickle cell disease is often fragmented, spanning multiple providers and often multiple institutions. This results in many persons with sickle cell disease not having a medical home that coordinates their care. A patient-centered medical home is an approach to providing comprehensive primary care for children, adolescents and adults that is patient- and family-centered, comprehensive, coordinated, accessible and committed to quality and safety.<sup>12</sup> The location of the medical home for individuals with sickle cell disease may vary based on patient and family preferences, and proximity to primary care and specialty care providers.<sup>13,14</sup> One study highlighted that many children with sickle cell disease did not have care that met the standards for a patient-centered medical home.<sup>15</sup> Additional literature has also shown that patients who receive comprehensive care had fewer emergency department visits and hospitalizations.<sup>16</sup> Coordination between primary and specialty care is crucial to the provision of high quality care for patients with sickle cell disease, as the lack of regular ambulatory care may lead to increased health care utilization in acute care settings (including increased reliance on the emergency department, particularly among transition-age youth (ages 12-25) and adults<sup>17</sup>) as well as missed opportunities for preventive care. Lack of outpatient hematology follow up after hospital discharge is a known risk factor for 30 day readmission among individuals with sickle cell disease.<sup>18</sup>

One particularly important area of care coordination is the promotion of chronic illness self-management, which is crucial to improving outcomes for children and adults with sickle cell disease.<sup>19</sup> Patients and families have a central role in managing their own or their child's health. Engaging in healthy behaviors such as adhering to prescribed medications, eating a nutritious diet, drinking plenty of fluids, staying active, avoiding extreme temperatures and managing stress levels can lead to fewer instances of complications such as pain crises, and thus improve outcomes and overall quality of life. Knowing how to manage mild complications at home and when to appropriately seek health care also contributes to improved quality of life and may lead to lower health care utilization costs.

Improvements in the realm of care coordination are essential and will require both leveraging pre-existing relationships within networks and developing new relationships to expand and extend clinical and psychosocial services. In turn, these efforts will improve processes to increase the speed and ease with which patients are able to access health services, as well as address some of the psychosocial issues that are often seen in this population, including mental health issues, unemployment, and homelessness. Ultimately, improvements in the coordination of care across multiple systems and networks and in the provision of primary and specialty care will enhance the quality life of individuals with sickle cell disease.

## RECOMMENDATIONS:

1. Develop an individualized care plan collaboratively with patient and/or family to facilitate communication of patient's current treatment plan.
2. Develop health maintenance tool to monitor and track patients' preventive screenings and vaccinations related to their care. Patients can be contacted to come in for requisite screenings and/or vaccinations.
3. Develop process for co-management between primary care provider and specialty provider; specifically outline which provider is responsible for each element of a patient's care.
4. Incorporate care team huddles or meetings each week to review patients' charts and/or care coordination tool and plan care that needs to be provided at upcoming medical visits.
5. Share tools such as health passports or patient diaries with patients that can be used to record, track and manage their treatment and care. Patients can also use this to coordinate care among clinicians.
6. Consider use of community health workers or patient navigators to assist with coordinating patient care.
7. Consider providing patients with self-Management training such as the Stanford University Chronic Disease Self-management Program (CDSMP).

**TABLE 2:** High-leverage changes and resources tested by grantee networks in medical home/care coordination

Change Idea	What is it? (Definition) Why do we use it? (Rationale)	Resources
Individualized care plans	A medical summary that is a shared document including the patient/family perspective and values. This summary includes a listing of patient demographic information including patient and family (if applicable) contact information, sickle cell genotype, past medical and surgical history, medications, medication and food allergies, baseline lab results, pain management plan (home, emergency department, inpatient setting), treatment algorithms for pain, asthma action plan, provider information (primary care provider and sickle cell team members), pharmacy information, health insurance information, and disability level (if applicable).	<a href="#">Illinois SCDTDP Patient Needs Assessment form</a> <a href="#">Ohio SCDTDP electronic health record tool (sickle cell disease-specific EPIC template "SMART Phrase)</a> <a href="http://www.medicalhomeinfo.org/how/care_delivery/#care">http://www.medicalhomeinfo.org/how/care_delivery/#care</a>
Health maintenance tracking tool	This tool provides a strategy for providers to track the care that patients receive and ensure that patients are up to date with their preventative care (e.g. screenings and vaccinations). This tool could be a paper-based checklist or embedded in the electronic medical record. This tool can be used during pre-clinic team meetings or huddles which is when the care team assembles at a predetermined time to look ahead on the schedule and anticipate the needs of the patients coming to the clinic on a particular day.	<a href="#">Illinois SCDTDP adult patient tracking log, care coordination checklist and screening tool</a> <a href="#">New York SCDNBSP Well Sickle Checklist</a> <a href="#">New Jersey Health Maintenance Checklist</a>
"Health passport"/ patient diary	<p>Patient-centered tool that includes a patient's medical history and contact information for care providers used to facilitate communication between patient and providers.</p> <p>Patients can track their symptoms and interventions at home and use the data to consult with providers.</p>	<a href="#">New York SCDNBSP patient event diary</a> <a href="#">Ohio SCDTDP electronic health record tool (sickle cell disease-specific EPIC template "SMART" Phrase)</a>



Change Idea	What is it? (Definition) Why do we use it? (Rationale)	Resources
Patient navigators/ community health workers	A patient navigator or community health worker is a member of the healthcare team who helps patients navigate and understand the healthcare system and get timely care. Navigators help coordinate patient care and can improve access to health care and social services such as insurance, housing, and employment.	<a href="#">Maryland SCDTDP (Urban Health Institution Community Health Worker program and the iHOMES program)</a> <a href="#">Colorado SCDTDP Patient Navigators</a>
Patient self-management training	Self-management programs like the Stanford University Chronic Disease Self-Management Program train patients to deal with problems related to living with a chronic disease, appropriate exercises to enhance flexibility and endurance, use of medications, communication with health care providers and evaluating new treatments. Such programs build confidence, empowerment and decision-making skills among patients	<a href="#">Stanford Chronic Disease Self-Management Program</a> <a href="#">New York SCDNBSP Handout: Well Sickle Care Screening - Why needed?</a> <a href="#">California SCDTDP Handout: What is Comprehensive Care in Sickle Cell Disease?</a> <a href="#">California SCDTDP Surveys (Barriers to Care, Iron Overload, Chelation Adherence, Improving School Success)</a> <a href="#">Tennessee SCDTDP online training modules</a>
Provider education to enhance patient self-management	The ACCEPT program (Advancing Communication and Care by Engaging Patients in Training) trains providers to integrate self-management support strategies (such as goal-setting) into routine clinical care.	<a href="#">Ohio SCDTDP and Ohio SCDNBSP's ACCEPT Training Materials, including overview and follow-up</a>

## SCREENING AND FOLLOW UP

Early studies documented that the early administration of penicillin prophylaxis reduced the incidence of pneumococcal infections by 84 percent and reduced mortality from such infections in children with sickle cell disease.<sup>20</sup> This finding provided the rationale for newborn screening and early diagnosis (in the newborn period) to ensure prompt treatment of affected individuals.<sup>20</sup> The result of screening performed in the neonatal period has immediate implications for the infant found to have the disease, but also longer-term implications for both the child and other family members, such as the ongoing need for genetic counseling and education.<sup>21</sup>

Only since May 1, 2006, have all U.S. states and the District of Columbia required and provided universal newborn screening for sickle cell disease, which also identifies sickle cell trait, despite a national recommendation to this effect in 1987.<sup>22,23</sup> Each state has developed a newborn screening program that meets the needs and resources of the state. For sickle cell disease and sickle cell trait, some states have well-developed follow-up programs in which nurses, program specialists or community-based organizations contact families of infants with positive newborn screening results and, as necessary, arrange confirmatory testing and follow up with specialists and genetic counselors.<sup>24</sup> Other states rely on the primary care provider to arrange for confirmatory testing, provide education to parents and refer patients to specialists.<sup>25</sup> Variation also exists in the process of screening individuals who are not screened as infants including pregnant women and immigrants.

NICHQ encourages organizations involved in the care of individuals with sickle cell disease to partner across their communities to incorporate screening genetic counseling and education into their outreach activities. This will expand the reach to diverse populations such as recent immigrants who were not screened in the newborn period.

## RECOMMENDATIONS:

1. State newborn screening programs should communicate results to patients or families and primary care providers.
2. Parents or caregivers of patients with confirmed diagnosis of sickle cell disease should receive genetic education about sickle cell disease.
3. Patients with confirmed diagnosis of sickle cell disease should be seen by a hematologist within three months of diagnosis.
4. Patients with confirmed diagnosis of sickle cell disease (SCD-SS and SCD-Sbeta zero thalassemia) should have prophylactic antibiotics initiated within three months of diagnosis to prevent invasive pneumococcal disease.
5. Patients with SCD-SS and SCD-Sbeta zero thalassemia who are younger than five years of age should be prescribed prophylactic antibiotics to prevent invasive pneumococcal disease.
6. Offer genetic education to individuals of reproductive age with sickle cell disease and sickle cell trait to allow for informed decision making. Consider developing electronic medical record prompts and other methods to alert providers that genetic counseling is needed during adolescence.
7. Consider conducting community outreach activities (such as health fairs, public service announcements, or social media posts) to encourage screening for sickle cell disease and sickle cell trait for individuals who were not screened in the newborn period.

**TABLE 3:** High-leverage changes and resources tested by grantee networks in screening and follow up

Change Idea	What is it? (Definition) Why do we use it? (Rationale)	Resources
Educational and counseling strategies	Educational and counseling strategies include providing counseling and education over the phone, group clinic visits for newborns with sickle cell disease, and electronic health record prompts to remind providers to counsel sickle cell disease patients. Education entails information about genetics of sickle cell disease, managing pain crises and other sickle cell related complications, reproductive implications and health maintenance strategies. Education should be age-appropriate and occur throughout the lifespan for individuals with sickle cell disease and trait.	<a href="#">Missouri SCDTDP Screening and Trait Counseling Education Booklet and Presentation</a> <a href="#">Tennessee SCDTDP Genes for Teens and Genes for Parents of Children with Sickle Cell Disease</a> <a href="#">Massachusetts SCDNBSP Parent's Guide to Sickle Cell Disease</a>
Pre- and post-tests	Questionnaires to assess patient/family knowledge before and after counseling. Administer pre-tests before offering education and post-tests immediately after as well as 3-6 months later to assess retention of knowledge.	<a href="#">Illinois SCDTDP pre- and post-tests</a> <a href="#">Illinois SCDNBSP pre- and post-tests</a>
Sickle cell trait toolkit	This toolkit was developed by grantee network teams to help providers counsel individuals and families recently diagnosed with sickle cell trait. Toolkit provides educational materials about sickle cell trait and sickle cell disease that can be reviewed by families on a periodic basis.	<a href="#">Screening Affinity Group Sickle Cell Trait Counseling Resource Packet</a>

## TRANSITION OF CARE

Because of great strides over the past few decades in care for individuals with sickle cell disease, these individuals are now living longer, transitioning from pediatric to adult care as they grow older. As patients transition from pediatric care to adult care, they experience a variety of challenges including leaving a familiar provider and environment, being seen by a provider who may not have knowledge of sickle cell disease, establishing independence from caregivers, and having adequate health insurance.<sup>26</sup> Multiple factors may contribute to high mortality during the period immediately following transition from pediatric to adult care including disease progression, lack of routine care and adherence to treatment.<sup>27</sup> In addition to increased mortality, young adults with sickle cell disease utilize emergency care services more often and have less frequent care maintenance visits during the transition years.<sup>17</sup> Planned and coordinated transition from pediatric care to adult care is critical in ensuring no interruption in care continuity and improving health outcomes and overall quality of life of individuals with sickle cell disease.

## RECOMMENDATIONS:

1. Develop a registry or listing of transition age youth in sickle cell program.
2. Establish a transition clinic/program to facilitate transition to adult care for patients 12 years and older that includes an agreed-upon transition policy posted in a visible place (e.g., waiting room, exam room, office).
3. Incorporate individual transition readiness assessments or checklists to prepare patients for transition of care.
4. Connect families, in advance of transition, with community and social services for planning and care coordination.
5. Consider scheduling a joint visit between the patient, pediatric hematologist or physician and adult hematologist or physician prior to transfer of care.

**TABLE 4:** High-leverage changes and resources tested by grantee networks in transition of care

Change Idea	What is it? (Definition) Why do we use it? (Rationale)	Resources
Transition clinic	A transition clinic/program allows providers, patients and families to prepare for the transfer of care from pediatric to adult settings. Ideally, the process of preparing for transition to adult care begins in early adolescence. In developing a clinic, the first step is developing a transition policy. Clinics/programs must develop a method (e.g. registry) of tracking and monitoring transitioning patients, assessing readiness, and transferring care. Transfer is complete if the patient continues to attend visits with an adult provider.	<a href="#">New Jersey SCDTDP Transition Policy</a>
Transition readiness assessment	Tools used to assess adolescents' knowledge and self-efficacy in various knowledge domains including medical, cognitive, emotional, psychosocial, and academic. Skills assessed vary by age and patients should demonstrate increased autonomy over time. Assessments should be administered at the start of the transition period and throughout the process. Results should be used to inform the education individual patients receive during the transition process.	<a href="#">California SCDTDP Transition Intervention Program –Readiness for Transition Assessment</a> <a href="#">Tennessee SCDTDP Readiness Assessment for Academic, Emotional, Medical and Psychosocial domains</a> <a href="#">New Jersey SCDTDP Autonomy Preparation Questions</a> <a href="#">Colorado SCDTDP Patient Activation Assessment</a> <a href="#">Colorado SCDTDP Changing Roles Assessment and Action Plan</a>
Sickle cell disease specific transition curriculum	The comprehensive curriculum covers all ages of the transition period (12-21 years of age) and includes recommendations of educational content for providers, patients and parents. The curriculum is organized into three main sections by age group, and each age group consists of three domains: medical, social, and academic. Use of the curriculum will ensure that all topics are covered throughout the transition planning process. Each domain includes guidelines for topics, suggested methodology, and techniques to measure efficacy. The curriculum can be used as a resource in both the medical and the community setting, and would be especially effective in organizing the work in partnerships.	<a href="#">Transition Affinity Group Sickle Cell Disease Transition Curriculum</a>

## HYDROXYUREA

Hydroxyurea is the only therapy approved for sickle cell disease by the Food and Drug Administration.<sup>28,29</sup> This medication results in a decline in sickle cell-related complications such as pain crises, acute chest syndrome and associated emergency department visits and hospitalizations.<sup>30</sup> By reducing the frequency of these complications of sickle cell disease, hydroxyurea can improve the quality of life for patients.<sup>31,32</sup> Hydroxyurea has been found to lower the costs associated with care for patients with sickle cell disease. While outpatient costs have been found to be higher, they are outweighed by the savings from fewer hospitalizations.<sup>30</sup>

Use of hydroxyurea varies greatly from region to region and provider to provider, highlighting a substantial opportunity to improve care by making hydroxyurea accessible to more patients.<sup>30</sup> One important barrier to the use of hydroxyurea is poor understanding of the clinical benefits, side effects, and long-term consequences of its use. Patients can obtain information from many diverse sources, some of which may be unreliable. Additional barriers to hydroxyurea use are focused at the health system level (e.g., insurance coverage) and provider level (e.g. knowledge, self-efficacy).

## RECOMMENDATIONS:

1. Discuss hydroxyurea (including side effects, benefits, and monitoring protocol) with patients with HbSS and Hb Sbeta zero Thalassemia and their families and incorporate patient preferences and values in decision making.
2. For adults with HbSS, treat with hydroxyurea if individual has three or more pain crises annually, has recurrent acute chest syndrome or severe pain impacting quality of life.
3. For infants older than nine months and children and youth, consider hydroxyurea treatment to prevent sickle cell-related complications.
4. Consider use of text/SMS messaging and other technologies to enhance adherence to hydroxyurea.

**TABLE 5:** High-leverage changes and resources tested by grantee networks in hydroxyurea

Change Idea	What is it? (Definition) Why do we use it? (Rationale)	Resources
Patient education	Videos, brochures, handouts and other information sources can be used with patients and families to convey information about hydroxyurea and clarify misconceptions about this treatment.	<a href="#"><i>Massachusetts SCDNBSP – Keeping you Healthy with Sickle Cell Disease</i></a> <a href="#"><i>New Jersey SCDTDP – The Best Hope for Sickle Cell (video)</i></a> <a href="#"><i>Tennessee SCDNBSP – Family Guide to hydroxyurea</i></a>
Decision support tools	Tools to guide patients and families through the process of evaluating the risks and benefits of hydroxyurea therapy can help facilitate the conversation and allow patients and families to feel more informed before making a decision.	<a href="#"><i>Massachusetts SCDNSBP hydroxyurea Dosing Guidelines</i></a>
Text/SMS messaging	Tool to send electronic message to patient's cell phone to remind patient to take medication (e.g. hydroxyurea).	<a href="#"><i>Tennessee SCDTDP- Scheduled Instant Messaging Over the Network (SIMON)</i></a>

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## GLOSSARY

## GLOSSARY

**Acute chest syndrome:** A complication of sickle cell disease in which a vaso-occlusive episode (see acute vaso-occlusive episodes) occurs in the pulmonary vasculature, the blood vessels of the lungs. Acute chest syndrome is characterized by fever, difficulty breathing and chest pain accompanied by a new pulmonary infiltrate on chest x-ray. This illness can be life-threatening.

**Acute vaso-occlusive episodes:** Also known as pain crises, these are the hallmark manifestation of sickle cell disease. These unpredictable episodes of pain can occur as early as 6 months of age and occur throughout the lifespan – in childhood, adolescence and adulthood. Pain crises are caused when sickled blood cells get stuck in small blood vessels and block the flow of blood to tissues. They are the primary reason that patients with sickle cell disease seek medical attention at health care facilities.

**Ambulatory care:** Health care services such as preventive care, subspecialty and/or acute care that are provided on an outpatient basis. These services may include a personal health care consultation, treatment, or intervention.

**Annual dilated eye exams:** Painless procedure in which drops are placed in the patient's eyes to dilate or widen the pupils to facilitate examination of eyes to look for vision problems and eye disease. This exam is a recommended screening for individuals with sickle cell disease due to the potential for the disease to cause vision loss.

**Change idea:** A specific idea for changing a process that can be tested to see if it results in improvement. Examples of change ideas include pain calculators for determining pain medication in the emergency department and text messages as a way to remind patients of upcoming appointments.

**Day hospital:** A hospital, or a specified area within a hospital, which provides an alternative to inpatient care for individuals with sickle cell disease. Services may include acute pain management, transfusions and/or primary care or subspecialty assessments depending on the location.

**Electronic Health Record/Electronic Medical Record:** A digital version of a patient's paper chart. These electronic charts are real-time, patient-centered records that are designed to make information available quickly and securely to authorized users.

**Genotype:** The alleles (different forms of a gene) which an individual has with respect to a particular characteristic. An individual inherits two alleles for each gene, one from each parent. For example, individuals with sickle cell anemia have inherited two copies of the gene for sickle cell hemoglobin. The major sickle cell genotypes are: Sickle cell anemia (HbSS) and sickle cell beta zero thalassemia, which usually are associated with a moderate to severe clinical course, and sickle-hemoglobin C disease (Hb SC) and sickle cell beta plus thalassemia, which are characterized by mild to moderate clinical severity.



**Glowcap®:** The function of this innovative device is a series of escalating reminders. The Glowcaps® fit on standard-sized pill bottles and a chip inside the cap monitors when the bottle is opened. At the scheduled time to take the medicine, the cap and a reminder light begin to glow. A gentle alarm sounds and the lights and sound alarm become more insistent if the pill bottle is not opened. If there is still no response, the cap can make a digital phone call or send a text message, depending on the patient's preferences. When the pill bottle is opened, the cap assumes the medicine has been taken and compiles a weekly and monthly report about adherence. These reports can be sent to patients, providers and a support person, by text or email, with the patient's permission. A simple push of a button inside the Glowcap® can be used for automatic prescription refills.

**Hemoglobin:** The protein in red blood cells that carries oxygen from the lungs to the rest of the body. In patients with sickle cell disease, a mutation in the hemoglobin gene produces slightly abnormal hemoglobin that can cause the red blood cells to become rigid and assume a sickle-like or crescent shape (whereas healthy red blood cells are round and flexible). The different genotypes of sickle cell disease (see genotype) result from differences in the exact mutation in the hemoglobin gene.

**Improvement advisor:** A project faculty member devoted to helping identify, plan, and execute improvement projects. Improvement advisors are specialists in the methods of improvement science and provide expert technical assistance to teams, assess data and provide constructive feedback to drive the testing and implementation of best practices.

**Individual pain action plans:** A written plan that individuals develop with their health care provider to help guide pain management at home and sometimes in acute care settings such as the emergency department. The plan lists pain medications and doses that have been previously effective for that individual. These plans may also include complementary strategies that patients can use on their own to manage pain, such as drinking water, applying heat pads and using distraction and/or guided imagery techniques. These plans also help to track pain control over time.

**Infusion center:** A setting where the clinical care provided pursuant to physician orders is managed and performed by nurses and registered pharmacists that are highly skilled in provision of infusion/specialty drug administration care of individuals with chronic medical conditions such as sickle cell disease and cancer.

**Intranasal fentanyl:** The intranasal administration of fentanyl, a fast-acting opiate pain medication. Intranasal fentanyl is administered as a squirt into the nose.

**Hydroxyurea:** The only therapy approved by the Food and Drug Administration for the treatment of sickle cell disease. It is an orally administered chemotherapeutic drug historically used to treat a number of diseases, including some cancers. In patients with sickle cell disease, hydroxyurea reduces the extent to which blood cells assume a sickle shape, reducing the occurrence of sickle cell disease-related complications such as pain crises and acute chest syndrome

**Medical home:** A medical home is not a building, house, hospital, or home healthcare service, but rather an approach to providing comprehensive primary care for children, adolescents and adults that is patient- and family-centered, comprehensive, coordinated, accessible and committed to quality and safety.

**Motivational interviewing:** Motivational interviewing is a client-centered, directive therapeutic style to enhance readiness for change by helping clients explore and resolve ambivalence.

**Non-random/special cause variation:** In improvement science this is variation that is not part of the system all the time but arises because of specific circumstances, such as a change idea being tested. Detecting special cause variation in a control chart signals that a change that a team is testing may be resulting in a change in performance.

**Oral Iron chelation:** Medication taken by mouth to remove excess iron likely due to frequent blood transfusions among individuals with sickle cell disease.

**Parenteral analgesia:** Pain medication administered through intravenous injection, through intramuscular injection or subcutaneously.

**Patient-controlled analgesia:** A method of pain control designed to allow the patient to administer pre-set doses of an analgesic (pain medication), on demand. The medications are most commonly administered using an intravenous analgesic infusion pump. Patient-controlled analgesia allows patients to control the timing of intravenous administration of their own pain medication resulting in faster alleviation of pain.

**Prophylactic penicillin:** The antibiotic penicillin given on a daily basis to children with sickle cell disease less than 5 years of age to prevent invasive pneumococcal infections such as blood stream infections.

**Run chart:** A graphical display of data plotted in some type of order, usually over time.

**Shewhart chart:** An extension of a run chart (see run chart) which contains upper and lower control limits (UCL and LCL) showing the expected variation in the measure. A Shewhart chart can be used to distinguish between variation in a measure of quality due to common causes (variation that is inherent in the system over time and affects everyone working in the system) and variation due to special causes (causes that are not part of the system all the time but arise because of specific circumstances, such as the testing of a change idea (see change idea). Shewhart charts are also known as control charts.

**Sickle cell disease:** A group of inherited blood disorders characterized by an abnormality in the oxygen-carrying hemoglobin molecule in red blood cells, which causes them to become rigid, sticky and sickle-shaped under certain circumstances. Sickle cells are also fragile, often dying early and causing a shortage of red blood cells resulting in chronic anemia. Sickle cell disease is caused by inheriting two genes for sickle hemoglobin, one from each parent. There are several different genotypes of sickle cell disease. The disease causes a variety of serious health complications including infection and stroke.

**Sickle cell trait:** An individual has sickle cell trait when he or she has inherited one gene for sickle hemoglobin and one gene for normal hemoglobin. An individual with sickle cell trait is a carrier for sickle cell disease, and can pass it on to a child, but usually does not have any of the symptoms of sickle cell disease.

**Standard order set:** Standard order sets are a group of medical orders used to standardize diagnosis and treatment for specific medical conditions such as sickle cell pain based on clinical practice guidelines. These order sets communicate best practices, reduce variation and potential for medical errors and enhance workflow. In this context, the order set standardizes the timeframes for triage, medication administration, and reassessment of pain with the goal of expediting patient care and decreasing delays in critical interventions such as administration of pain medication. Standard orders can be paper based or embedded in computerized physician order entry system in an electronic health record.

**Stanford University's Chronic Disease Self Management Program:** A program designed to help people with chronic diseases gain self-confidence and improve their ability to control their symptoms, better manage their health problems, and lead fuller lives. The program has a strong evidence base, with demonstrated improvements for a range of patient groups in exercise, active coping, symptom management, quality of life and communication with health care providers.

**Transcranial Doppler screening:** A non-invasive radiologic ultrasound test that measures the velocity of blood flow through the brain's blood vessels. Transcranial Doppler screening is typically performed between 2 and 16 years of age in sickle cell patients to identify those individuals most at risk for stroke so that they can receive treatment that lowers their risk of stroke.

**Transition age:** Transition planning process should begin at the age of 12. The transfer from pediatric to adult focused care ideally occurs between the ages of 18 and 21 yrs. The transfer planning process, patients are building capacity to the degree possible to effectively transition from pediatric to adult care.

## TECHNICAL METHODS

# TECHNICAL METHODS

## I. QUALITY IMPROVEMENT METHODS

### Interpretation and display of quality improvement data

Data collected were plotted on either a run chart or Shewhart chart. Both of these charts display data over time in graphical form and are used to evaluate the success of improvement efforts in an objective way. Run charts and Shewhart charts are utilized to study how a measure changes over time, and allows for the identification of both random and non-random patterns within the data. When random patterns are identified in the data there is little confidence that the change a team is testing is leading to improved results. When non-random patterns are identified, this is a “signal” of change in the measure. Time ordered data collected and analyzed in a run or Shewhart chart can determine statistical significance when other research focused tests (t-test, chi-square, F Test) are not appropriate. There are a series of probability-based rules used to interpret the data found in a run chart. When one or more of these rules are met there is evidence of non-random variation based on an alpha error of  $p < 0.05$ .<sup>1</sup>

## 2. CLIENT SURVEY DATA ANALYSIS METHODS

### Survey Content

The Individual Utilization Questionnaire assessed the following: demographics, income and educational status, type of disease, age at diagnosis, utilization of services, hospitalization, complications, and treatment status including antibiotics, hydroxyurea, transfusion, and counseling. These were collected based on patient self-report/parent report (in some cases data were confirmed by medical record). Immunization history was collected based on medical record. Analysis for change over time (longitudinal analysis) is presented based on matched baseline and follow-up data for 1,642 patients.

The two instruments used to assess quality of life for patients living with sickle cell disease, the SF-8 (the previous round of data collection used the longer version, the SF-36) and Pediatric Quality of Life Inventory (PedsQL), are 8-item and 15-item surveys, respectively.<sup>2,3</sup> These scales have been used in large population studies.<sup>4,5,6,7,8</sup> Both the SF-8 and the PedsQL provide a health profile that encompasses physical and emotional health. Specifically, the SF-8 provides summary scores for the following domains of health in adults: physical functioning, role limitations due to physical health, bodily pain, general health perceptions, vitality, social functioning, and role limitations due to emotional problems and mental health. The PedsQL measures health-related quality of life specifically in children and adolescents, through brief, practical, generic core scales such as performance in general physical health, emotional health, social skills, and in academics. It is administered to both parents and pediatric patients.

### Statistical Methods & Analyses

Data for this analysis were downloaded from an electronic data entry system, Research Electronic Data Capture (REDCap). REDCap is a secure, web-based application for building and managing online surveys. Files were downloaded in .csv format and analyzed using SAS 9.3 (SAS Institute Inc., Cary, NC). Trend data was plotted using STATA/SE (StataCorp. 2009. Stata Statistical Software: Release 10.1, College Station, TX: StataCorp LP).

Descriptive statistics were calculated for demographic characteristics. Frequencies and proportions were calculated for categorical variables such as gender, race, sex, ethnicity, health care utilization measures, and sickle cell disease type. Continuous variables such as age, age at diagnosis, hemoglobin levels were summarized by mean, median, standard deviation, minimum and maximum value. Outliers and possible data errors were detected for further formal statistical analysis. Differences between self-reported data vs. medical record data were also described (e.g., SCD type and hemoglobin levels from the Individual Utilization Questionnaire compared to medical records).

Tests for differences across site were analyzed using one-way ANOVA for continuous variables and chi-square test of independence for categorical variables. Fisher's exact test was reported for rare events (cell counts <5). Analyses of differences from baseline to follow-up are performed using paired t-tests for continuous variables and McNemar's chi-square for categorical variables.

Trend analysis was performed by generalized additive models (GAM) to estimate the average item-response of the study sample over time ("population-averaged" effects). The GAM tests whether the linear model is sufficient and is a test of trend for reported outcomes including hospitalizations and emergency department visits, hydroxyurea use, and hydroxyurea counseling. Generalized Estimating Equation (GEE) were performed in order to obtain a more formal p-value testing for association and in order to account for repeated measures. Family Poisson (log link) is applied to the analysis for continuous variables, while family binomial (logit link) is utilized for binary outcomes. Trend analysis was done, both un-weighted and weighted to account for number of observations per team. Raw data was plotted with each dot representing an outcome of interest (e.g. emergency department visit) for a particular day, and across time, during the study period. The same individual may have multiple dots on the graph if they had more than one emergency department visit during the study period. The trend line from the GAM is included in the graph.

The responses from the SF-36 and SF-8 Health Surveys and PedsQL surveys were reverse-scored on a scale of 0-100 with 100 representing the highest level of functioning possible. Only items from the PedsQL-23 item survey that are in common with the PedsQL 15-item survey were included in this analysis. Each domain is the mean of questions within that domain and Total is the mean of all questions. As suggested in the guidelines,<sup>9</sup> the denominator excluded missing values. The results were presented as mean scores with standard deviations.

PedsQL scores for this study were compared to those presented from other studies, based on a literature review. Comparisons scores for SF-36 and SF-8 were not provided because they were either not available for sickle cell disease patients (as is the case for SF-8) or were from international groups with small sample size (as is the case for SF-36).

### **Cross Sectional Analysis**

The last set of analyses compared data from the prior National Coordinating Center and the current National Coordinating Center. We have made the assumption that observations were independent. For categorical variables, Chi Square and Fisher's T test were used to describe the two populations and p values were calculated to explore whether outcomes differed significantly between the prior and the current NCC. A GEE Regression (outcome = timescale + NICHQ + timescale\*NICHQ) model was used to test hospitalization/ED visits over time. A p-value to assess for interaction between outcome and time was also calculated.

We considered P values <0.05 as statistically significant; all tests were two-sided.

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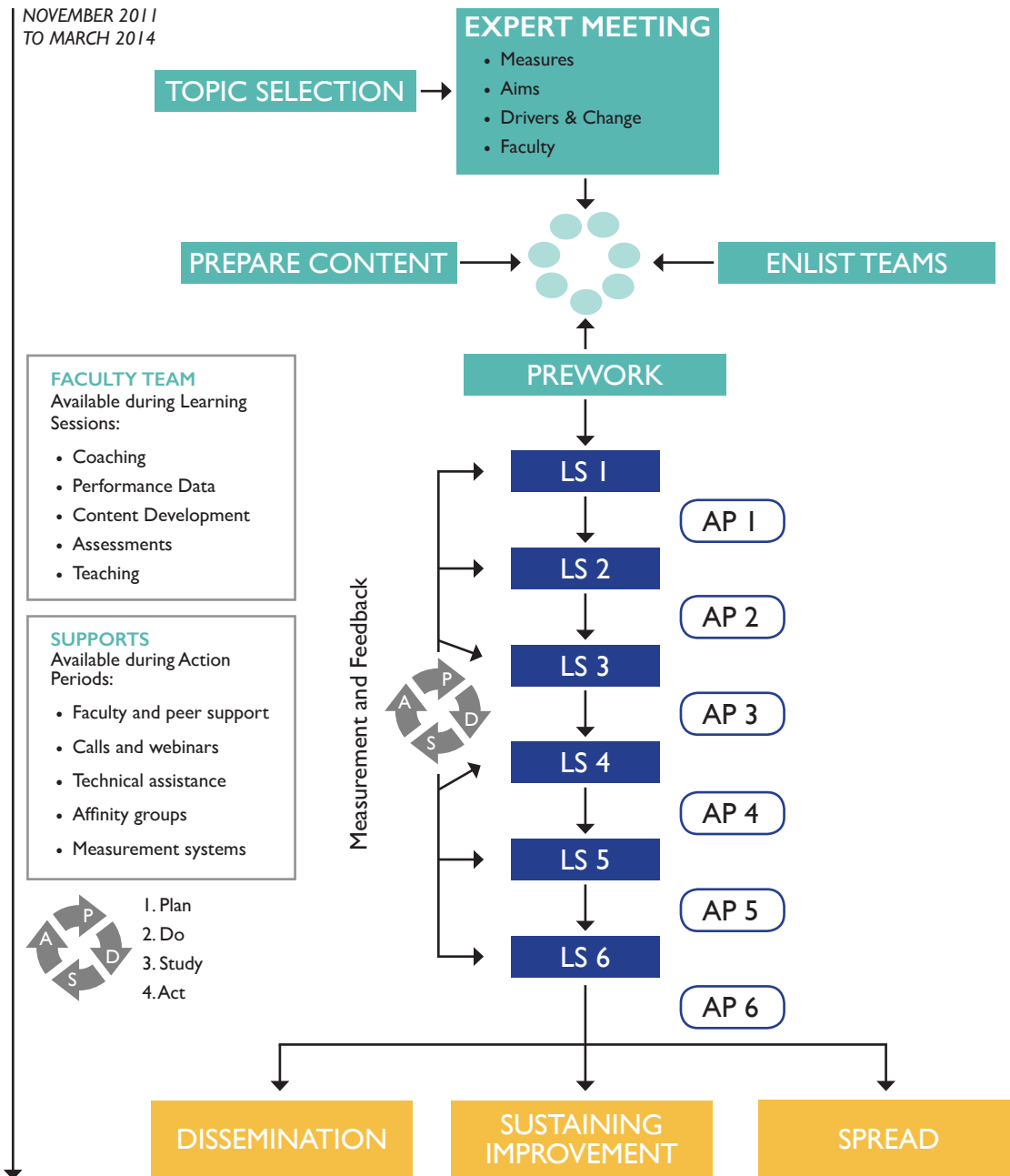
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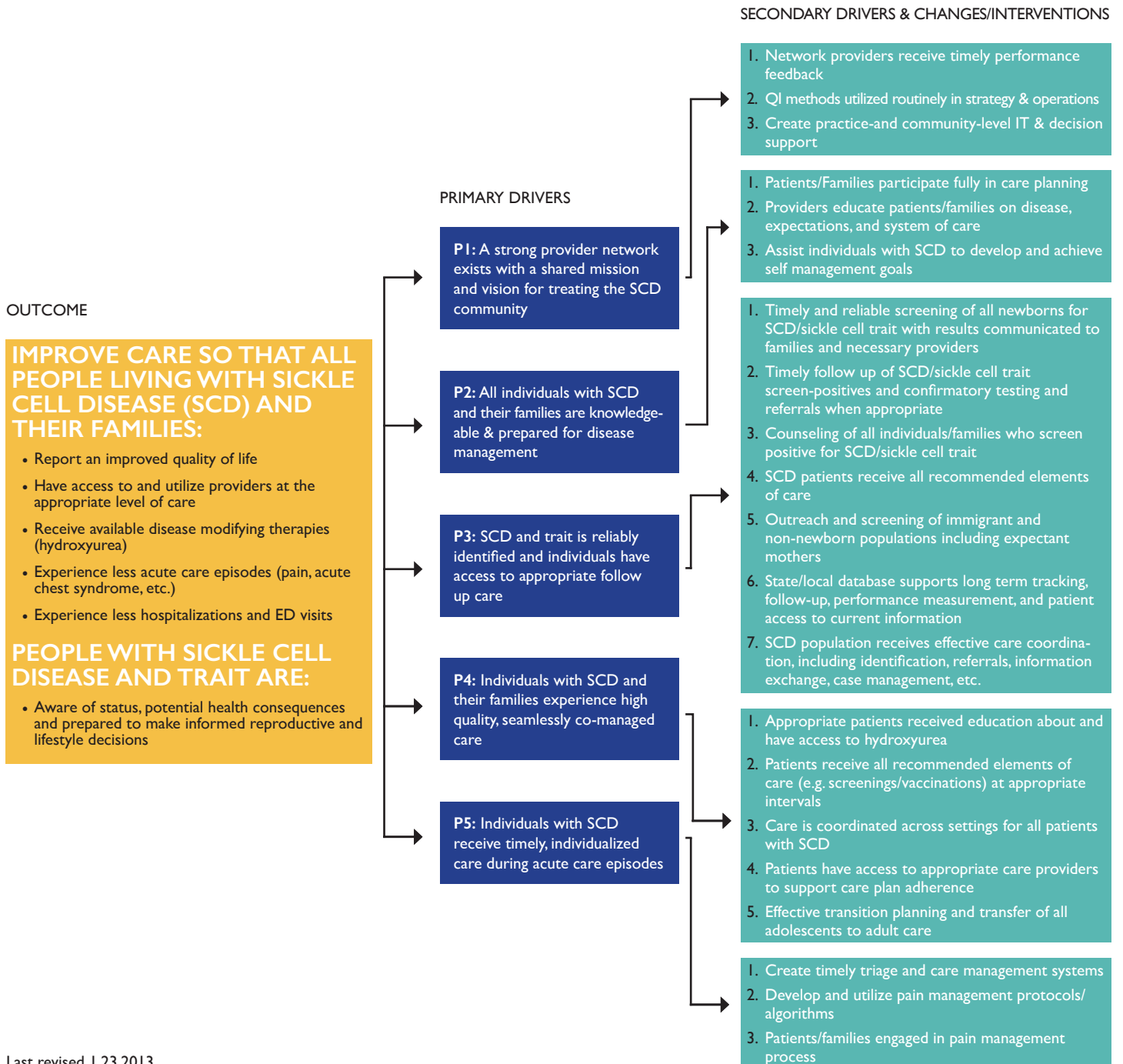
## ORGANIZING FRAMEWORKS



# ORGANIZING FRAMEWORKS



# DRIVERS FOR IMPROVING SICKLE CELL CARE



Last revised 1.23.2013

**TABLE 1:** Sickle Cell Disease Treatment Demonstration Program Expert Meeting Participant List


Name	Professional Domain	Position and Affiliation
Lennette Benjamin, MD	<i>Pain management</i>	Professor Emerita, Department of Medicine (Hematology), Montefiore Medical Center
Mauvereen Beverley, MD	<i>Adult primary care-specialty care interface</i>	Internal Medicine, Queens Health Network
David Brousseau, MD	<i>Emergency care</i>	Professor and Chief of the Section of Pediatric Emergency Medicine, Medical College of Wisconsin
David Bundy, MD, MPH	<i>Sickle cell disease and quality improvement</i>	Associate Professor of Pediatrics, Vice Chair for Quality and Safety, MUSC Department of Pediatrics Johns Hopkins University
Alissia Cofer	<i>Patient perspective</i>	Young adult consumer partner
W. Carl Cooley, MD	<i>Transition</i>	Chief Medical Officer, Crochted Mountain Foundation; Medical Director, Center for Medical Home Improvement
Michael DeBaun, MD, MPH	<i>Primary care-specialty interface and stroke prevention</i>	Professor of Pediatrics and Medicine J.C. Peterson Chair in Pediatric Pulmonology Director, Vanderbilt-Meharry Center for Excellence in Sickle Cell Disease
Tiffany Diers, MD (Ohio)	<i>SCDTDP grantee representative</i>	Associate Professor of Medicine, General Internal Medicine Division, College of Medicine University of Cincinnati
Kathryn Hassell, MD (Colorado)	<i>SCDTDP grantee representative</i>	Director, Colorado Sickle Cell Treatment and Research Center, University of Colorado, Denver
Carlton Haywood, Jr. PHD, MA	<i>Bioethics and sickle cell disease</i>	Core Faculty, Berman Institute of Bioethics, Core Faculty, Welch Center for Prevention, Epidemiology, and Clinical Research, Assistant Professor, Division of Hematology, The Johns Hopkins School of Medicine
Danita Johnson	<i>Parent perspective</i>	Parent partner
Kwaku Ohene-Frempong, MD	<i>Pediatric clinical leader (specialist-generalist for sickle cell disease)</i>	Director Emeritus, Comprehensive Sickle Cell Center, The Children's Hospital of Philadelphia
Janet Ohene-Frempong, MS	<i>Education and behavior change</i>	President of J. O. Frempong & Associates, Inc.
Suzette Oyeku, MD, MPH	<i>Pediatric primary care-specialty interface</i>	Associate Professor of Clinical Pediatrics, Associate Division Chief for Academic Affairs, Division of General Pediatrics Associate Director, Leadership, Engagement and Diversity Office Montefiore Medical Center, The University Hospital for Albert Einstein College of Medicine

**TABLE 2:** Sickle Cell Disease Newborn Screening Program Expert Meeting Participant List

Name	Professional Domain	Position and Affiliation
Maria del Pilar Aguinaga, MD	<i>Obstetrics and gynecology</i>	Professor; Department of Obstetrics and Gynecology, Associate Director, Sickle Cell Center, Meharry Medical College
Althea Grant, PhD	<i>Sickle cell trait</i>	Commander, US Public Health Service; Chief, Epidemiology and Surveillance Branch, Division of Blood Disorders, National Center on Birth Defects and Development
Katherine Harris	<i>State newborn screening program</i>	Project Manager, New York Mid-Atlantic Consortium for Genetics and Newborn Screening Services, New York State Genetic Service Program Director
Keith Hoots, MD	<i>National Heart, Lung, and Blood Institute</i>	Director, Division of Blood Diseases and Resources, National Heart, Lung, and Blood Institute, National Institutes of Health
Alissia Cofer	<i>Confirmatory testing</i>	Associate Hematologist/Oncologist, Children's Hospital & Research Center of Oakland
Carolyn Hoppe, MD	<i>Community-based organization representative</i>	Executive Director, Sickle Cell Disease Association of Illinois
Talana Hughes, MPH	<i>Pediatric hematologist</i>	Director, Sickle Cell Center at Emory; Children's Healthcare of Atlanta
Peter Lane, MD	<i>Pediatric hematologist</i>	Director, Sickle Cell Center at Emory; Children's Healthcare of Atlanta
Dennis McCullum	<i>Patient perspective</i>	Consumer partner (Illinois SCDTDP team)
Jelili Ojodu, MPH	<i>National Newborn Screening &amp; Genetics Resource Center</i>	Director, Newborn Screening and Genetics Association of Public Health
Lauren Raskin Ramos, MPH	<i>Association of Maternal &amp; Child Health Programs</i>	Director of Programs, Association of Maternal & Child Health Programs
Lynnie Reid	<i>Parent perspective</i>	Senior Project Manager, NICHQ
Charmaine Royal, PhD	<i>Genetic counseling</i>	Department of African & African American Studies, Duke Institute for Genome Sciences & Policy
Joseph Telfair	<i>Public health</i>	Professor, Public Health Research and Practice, University of North Carolina at Greensboro
Kusum Viswanathan, MD	<i>Emerging populations</i>	Vice Chair, Department of Pediatrics; Director, Division of Pediatric Hematology/Oncology, Brookdale University Hospital and Medical Center

**TABLE 3:** SCDTDP Oversight Steering Committee Members

Name	Professional Domain	Position and Affiliation
Efa Ahmed-Williams	<i>Patient perspective</i>	Founder, Destiny Despite Sickle Cell Disease
Alice Cohen, MD, FACP	<i>Hematology</i>	Commander, US Public Health Service; Chief, Epidemiology and Surveillance Branch, Division of Blood Disorders, National Center on Birth Defects and Development
W. Carl Cooley, MD	<i>Transition</i>	Chief Medical Officer, Crotched Mountain Foundation; Medical Director, Center for Medical Home Improvement
James Eckman, MD	<i>Hematology</i>	Professor Emeritus, Hematology & Oncology, Emory University School of Medicine
Jane Hankins, MD, MS	<i>Hematology</i>	St. Jude Faculty, Department of Hematology, St. Jude Children's Research Hospital
Carlton Haywood, Jr. PHD, MA	<i>Bioethics and sickle cell disease</i>	Core Faculty, Berman Institute of Bioethics, Core Faculty, Welch Center for Prevention, Epidemiology, and Clinical Research, Assistant Professor, Division of Hematology, The Johns Hopkins School of Medicine
Thomas Howard, MD	<i>Pediatric hematology</i>	Professor of Pediatrics Director, Hematology Section Co-Director UAB Comprehensive Sickle Cell Center, Children's Hospital of Alabama and University of Alabama Hospital
Talana Hughes, MPH	<i>Community-based organization representative</i>	Executive Director, Sickle Cell Disease Association of Illinois (SCDAI)
Chazeman Jackson, PhD, MA	<i>Minority health</i>	Health Sciences Advisor, Office of Minority Health, US Department of Health and Human Services
Peter Lane, MD	<i>Pediatric hematologist</i>	Director, Sickle Cell Center at Emory; Children's Healthcare of Atlanta
Kwaku Ohene-Frempong, MD	<i>Pediatric clinical leader (specialist-general sickle cell disease)</i>	Director Emeritus, Comprehensive Sickle Cell Center, The Children's Hospital of Philadelphia
Barry Zuckerman, MD	<i>Pediatrics</i>	Joel and Barbara Professor of Pediatrics and Public Health at Boston University School of Medicine, and Chief of Pediatrics at Boston Medical Center

	Primary Levels	Secondary levels	Example Contextual Factors of Secondary Levels
<p style="writing-mode: vertical-rl; transform: rotate(180deg);">CONTEXT influencing success of WISCH teams</p> 	EXTERNAL ENVIRONMENT	1. <i>External environmental factors</i>	<ul style="list-style-type: none"> <li>Political climate &amp; economic environment (e.g. insurance policies, funding for SCD-related work)</li> <li>Demographic and geographic factors (e.g., rural/urban; access to medical facilities), SCD awareness</li> </ul>
		2. <i>External environment's relationship to WISCH</i>	<ul style="list-style-type: none"> <li>External incentives/barriers to WISCH work</li> <li>External support (personnel, grant funding, political figures) to WISCH work</li> <li>Funder's goals, guidance, program objective, available program funds</li> <li>Priority of WISCH (topic/aims) to broader environment</li> </ul>
	NETWORK (organizations)	1. <i>Network support for QI</i>	<ul style="list-style-type: none"> <li>Level of support and incentives for QI activities within your network organizations</li> <li>Staff knowledge, training, and uptake of QI methods</li> </ul>
		2. <i>Network relationship to WISCH</i>	<ul style="list-style-type: none"> <li>Network (non-project team ) leadership/buy-in for WISCH activities</li> <li>Fit of WISCH into network organizations' mission/work priorities</li> </ul>
		3. <i>Network culture and engagement</i>	<ul style="list-style-type: none"> <li>Structure and dynamics of participating sites (e.g. types of organizations, partnerships)</li> <li>Culture values teamwork, communication, improvement</li> <li>Enthusiasm about SCD-related work; engagement of community members and SCD Partners</li> </ul>
		4. <i>Network capacity and infrastructure</i>	<ul style="list-style-type: none"> <li>Leadership buy-in and facilitation of project</li> <li>Information system to pull and share data across networks (e.g. EMR, data collection processes)</li> <li>Adequate financial support, resources, time for project</li> </ul>
	COLLABORATIVE	1. <i>WISCH/NICHQ collaborative personnel</i>	<ul style="list-style-type: none"> <li>Faculty expertise , engagement, and QI support (e.g. Lanetta Jordan, Suzette Oyeku, Bill Adams)</li> <li>Individualized NICHQ staff support (e.g. site leads, monthly check-in calls); availability for guidance</li> <li>Project partnerships (e.g. SCDA, BMC, Community Catalyst, Family Voices); SCD expertise - NICHQ's capacity, experience; NICHQ staff turnover and internal transitions</li> </ul>
		2. <i>WISCH collaborative structure and systems</i>	<ul style="list-style-type: none"> <li>Sequencing of collaborative; quality of key documents, QI measures</li> <li>Infrastructure and systems for collaboration (e.g. affinity groups, activities, data systems)</li> <li>OMB/IRB requirements and impact on work; ILab and RED-Cap</li> <li>Clarity of WISCH aims; alignment with team's original project aims detailed in grant application</li> <li>Addition of NBSP teams to project after TDP teams</li> </ul>
	QI TEAM	1. <i>Composition</i>	<ul style="list-style-type: none"> <li>Previous QI experience among team members</li> <li>Having an engaged physician, consumer, and data manager</li> <li>Team member turnover or growth, staff changes</li> </ul>
		2. <i>Team Leadership (For WISCH: Team Lead or PI)</i>	<ul style="list-style-type: none"> <li>Level of power/influence of team lead; experience with SCD-related work/expertise on SCD/SCT</li> <li>Level of commitment to project; engagement in a variety of content and project areas</li> <li>Open communication and sharing of data (within and across teams)</li> </ul>
		3. <i>Engagement</i>	<ul style="list-style-type: none"> <li>Attendance at project events, completion of activities, frequency of meetings; involvement of all team members; engaging family/patients</li> <li>Participation in collaborative discussions; interaction with other teams; work in Affinity Groups</li> <li>Key players are involved in both collaborative and work on the ground</li> </ul>
		4. <i>Cohesion</i>	<ul style="list-style-type: none"> <li>Understanding of team roles</li> <li>Agreement on project goals</li> <li>Familiarity with, and respect for, other team members; open &amp; honest communication streams</li> </ul>

Contextual Factor Levels (avg across sublevels)	MA NBSP	St. Jude TDP	PA SCD TDP/NBS	New Jersey	California SCD TDP	IL NBSP	Ohio Valley SCNetwork	New York	CO TDP	IL TDP
External Environment	3.50	3	3.50	3	3	1.50	2	1.50	2.50	1.50
Network	5.00	4.50	3.88	3.75	2.50	3.50	2.75	2.25	3.75	1.75
Collaborative	2.5	3	3.5	3.5	4.5	5	4.5	4	2	2.5
QI Team	4.75	5.00	4.50	4.50	4.25	4.25	2.75	4.25	3.67	2.25
Team Average (across all levels)	3.94	3.88	3.84	3.69	3.56	3.56	3.00	3.00	2.98	2.00

SCALE FOR LEVELS



# MEASUREMENT BANK



# MEASUREMENT BANK

## Levels of Contextual Factors Influencing WISCH Team Success

Focus Area	Measure ID	Measure	Operational Definition (Numerator & Denominator or other)
Healthcare Use/ Outcomes	Hemecare6	<ul style="list-style-type: none"> <li>Average number of hospital stays for SCD- related pain per SCD patient in the past 12 months</li> </ul>	<p><b>Numerator:</b> Count of sickle cell pain related hospital admissions by sampled SCD patients in the past 12 months</p> <p><b>Denominator:</b> Count of sampled SCD patients</p> <p>NOTE:</p> <ul style="list-style-type: none"> <li>Sickle cell pain described as patient's experience of "sudden onset of pain in the low back or in one or more joints or one of the extremities. The pain may be localized or migratory and is continuous and throbbing."</li> <li>Some patients may be initially admitted for management of sickle cell pain and develop secondary acute chest syndrome - those patients could be included in this sample.</li> <li>Does not apply to hospitalizations for fever management or acute chest syndrome, surgeries or other indications for admission.</li> <li>Does not include children less than 2 yrs of age who are admitted for febrile illness evaluation.</li> <li><b>All teams should collect this measure regardless of focus area.</b> We request that you sample at least 80% of the patients in the target population for your network's improvement work and that you follow and report on the same patients each month.</li> <li>Data for this measure should be submitted through the <i>Quarterly Data Template</i>.</li> </ul>
	Hemecare7	<ul style="list-style-type: none"> <li>Average number of ED visits for SCD- related pain per SCD patient in the past 12 months</li> </ul>	<p><b>Numerator:</b> Count of sickle cell pain-related ED visits by sampled SCD patients in the past 12 months</p> <p><b>Denominator:</b> Count of sampled SCD patients</p> <p>NOTE:</p> <ul style="list-style-type: none"> <li>Does not apply to ER visits for fever management or acute chest syndrome.</li> <li>Does not include children less than 2 yrs of age who are seen and evaluated for febrile illness.</li> <li><b>All teams should collect this measure regardless of focus area.</b> We request that you sample at least 80% of the patients in the target population for your network's improvement work and that you follow and report on the same patients each month.</li> <li>Data for this measure should be submitted through the <i>Quarterly Data Template</i>.</li> </ul>
Increased proportion of patients on HU	Hemecare5	<ul style="list-style-type: none"> <li>Percent of SCD patients <math>\geq 24</math> months of age currently taking hydroxyurea therapy</li> </ul>	<p><b>Numerator:</b> Count of SCD patients <math>\geq 24</math> months of age as of the last day of the 3 month measurement period who are candidates for hydroxyurea and currently on hydroxyurea</p> <p><b>Denominator:</b> Count of SCD patients <math>\geq 24</math> months of age as of the last day of the 3 month measurement period who are candidates for hydroxyurea</p> <p>NOTE:</p> <ul style="list-style-type: none"> <li>WISCH program considers eligible candidates as those patients who are <math>\geq 24</math> months of age with HbSS and Hb Sbeta zero thalassemia regardless of disease severity. WISCH team recognizes organizations may use other eligibility criteria for HU that are based on clinical symptoms and/or disease severity. Please define any additional eligibility criteria your organization is using in column "I" of the HU tab of the <i>Quarterly Data Template</i>.</li> <li>For patient to be currently taking HU, they should have a record of an active prescription. Confirmation that patient is actually ingesting hydroxyurea is NOT needed to be included in the count for this metric</li> <li>This measure should be collected every 3 months and should include the total number of patients in your target population that your organization(s) consider eligible for HU. Your target population should include all patients served by your organization or network of organizations, but should not include all possible patients in your state with SCD. The target population is limited to those patients treated by your organization(s), whom you know of, have provided care to, etc.</li> <li><b>All teams should collect this measure regardless of focus area.</b></li> <li>Data for this measure should be submitted through the <i>Quarterly Data Template</i>.</li> </ul>

Focus Area	Measure ID	Measure	Operational Definition (Numerator & Denominator or other)
Improved ED Care	ED4	<ul style="list-style-type: none"> <li>Average time from triage to administration of parenteral analgesic for SCD patients presenting at the ED with acute pain</li> </ul>	<ul style="list-style-type: none"> <li>Mean in minutes of the interval from triage to administration of parenteral analgesic for SCD patients presenting at the ED with acute pain</li> </ul>
	ED5	<ul style="list-style-type: none"> <li>Percent of SCD patients presenting at the ED with acute pain who had pain reassessed within 30 minutes of administration of the first dose of parenteral analgesic</li> </ul>	<p><b>Numerator:</b> Count of SCD patients presenting to the ED in the measurement month who presented with pain, who received parenteral analgesic, and had pain reassessed within 30 minutes of initial parenteral analgesic administration</p> <p><b>Denominator:</b> Count of SCD patients presenting to the ED in the measurement month who presented with pain and who received parenteral analgesic</p> <p>NOTE:</p> <ul style="list-style-type: none"> <li>Pain assessment must be performed using an age- appropriate pain scale</li> </ul>
Medical home/ Enhanced Care Coordination	Medhome1	<ul style="list-style-type: none"> <li>Percent of SCD patients with an evaluation with a hematologist or sickle cell specialist documented within the past 12 months</li> </ul>	<p><b>Numerator:</b> Count of SCD patients with documented evaluation within 12 months of the last day of the measurement month.</p> <p><b>Denominator:</b> Count of SCD patients.</p> <p>NOTES:</p> <ul style="list-style-type: none"> <li>Sickle cell specialists include hematologist, nurse practitioner or physician assistant specializing in sickle cell care</li> <li>An evaluation' should include (1) review of medical history, (2) physical examination, (3) complete blood cell count and pulse oximetry</li> <li>Patients without a visit in the past 12 months, or lacking documentation are not included.</li> <li>The hematology or specialist visit must have been completed - referral alone is not sufficient for inclusion</li> <li>Telemedicine encounters are included</li> </ul>
	Medhome2	<ul style="list-style-type: none"> <li>Percent of adults with SCD <math>\geq</math> 18 yrs who had all recommended elements of care within the past 12 months</li> </ul>	<p><b>Numerator:</b> Count of SCD patients <math>\geq</math>18 yrs who had all of the following elements of care documented within 12 months of the last day of the measurement period: 1) screening for high blood pressure, 2) screening for depression, 3) ophthalmologic exam</p> <p><b>Denominator:</b> Count of SCD patients 18 yrs and older as of the last day of the measurement period.</p>
	Medhome3	<ul style="list-style-type: none"> <li>Percent of SCD patients <math>\geq</math> 16 years seen in the past month with a transition plan to adult care</li> </ul>	<p>Numerator: Count of SCD patients <math>\geq</math>16 years at the time of their most recent visit who had a current transition plan. Include patients whose plan was completed during the visit.</p> <p>Denominator: Count of patients <math>\geq</math>16 years with visits in the measurement month.</p> <p>NOTE: Transition plan could include the following elements:</p> <ul style="list-style-type: none"> <li>A written summary of the medical history (history of complications, status of recommended screenings; vaccinations, current medications, treatments)</li> <li>Patient's readiness to self-manage his or her health care</li> <li>Steps needed for a successful transition</li> </ul>
	Medhome4	<ul style="list-style-type: none"> <li>Percent of SCD patients with a documented primary care provider with whom the patient has completed at least 1 primary care visit within the past 12 months</li> </ul>	<p><b>Numerator:</b> Count of patients with a documented primary care provider with whom the patient has completed at least 1 primary care visit within the past 12 months</p> <p><b>Denominator:</b> Count of SCD patients</p>
	Medhome6	<ul style="list-style-type: none"> <li>Percent of SCD patients who have had a written individual care plan in past 12 months</li> </ul>	<p><b>Numerator:</b> Count of SCD patients with documented individual care plan within the past 12 months</p> <p><b>Denominator:</b> Count of SCD patients</p> <p>NOTE: Individual care plan could include:</p> <ol style="list-style-type: none"> <li>current medications,</li> <li>pain management plan,</li> <li>fever management plan,</li> <li>current blood counts (hemoglobin/hematocrit)</li> <li>asthma action plan if applicable</li> </ol>
	Medhome7	<ul style="list-style-type: none"> <li>Percent of SCD patients who have a written individual care plan that was reviewed with the patient during the current visit</li> </ul>	<p><b>Numerator:</b> Count of SCD patients with SCD visit during the measurement month with documented Individual care plan that was reviewed with the patient</p> <p><b>Denominator:</b> Count of SCD patients with SCD visit in the measurement month</p>

Focus Area	Measure ID	Measure	Operational Definition (Numerator & Denominator or other)
Medical home/ Enhanced Care Coordination cont.	Medhome8	<ul style="list-style-type: none"> <li>Percent of SCD patients &lt;18 years who are up to date with all recommended vaccinations</li> </ul>	<p><b>Numerator:</b> Count of SCD patients &lt;18 yrs as of the last day of the measurement period who are up to date with the following vaccinations: 1) PCV7 / PCV13/Prevnar, 2) PPV23/Pneumovax, 3) Meningococcal (MCV4 or MPSV4), 4) Haemophilus influenza (HIB), 5) annual influenza</p> <p><b>Denominator:</b> Count of SCD patients &lt;18 yrs as of the last day of the measurement period.</p> <p><b>NOTE:</b></p> <ul style="list-style-type: none"> <li>Please refer to the CDC immunization schedule and the catch-up immunization schedule for details <a href="http://www.cdc.gov/vaccines/recs/schedules/">http://www.cdc.gov/vaccines/recs/schedules/</a>.</li> <li>You do not need to document child is up to date on all childhood vaccinations only vaccines listed above</li> </ul>
	Medhome9	<ul style="list-style-type: none"> <li>Percent of SCD patients ≥18 years who are up to date with all recommended vaccinations</li> </ul>	<p><b>Numerator:</b> Count of SCD patients ≥18 yrs who are up to date with the following vaccinations: 1) PCV7 / PCV13/Prevnar, 2) PPV23/Pneumovax, 3) Meningococcal (MCV4 or MPSV4), 4) Haemophilus influenza (HIB), 5) annual influenza 6) Hepatitis B</p> <p><b>Denominator:</b> Count of sampled SCD patients ≥18 yrs as of the last day of the measurement period.</p> <p><b>NOTE:</b></p> <ul style="list-style-type: none"> <li>You do not need to verify all vaccinations received prior to 18 yrs of age; only vaccines listed above</li> </ul>
	Hemecare4	<ul style="list-style-type: none"> <li>Percent of SCD patients between ages 2-16 years who received a transcranial doppler within the past 12 months</li> </ul>	<p><b>Numerator:</b> Count of SCD patients ≥24 months and ≤16 yrs who received transcranial doppler screening within the past 12 months.</p> <p><b>Denominator:</b> Count of SCD patients 2-16 yrs as of the last day of the measurement period.</p> <p><b>NOTE:</b></p> <ul style="list-style-type: none"> <li>Individuals with HbSS and Hb Sbeta zero Thalassemia are recommended to receive transcranial Doppler screening</li> <li>If sampled patient had more than one transcranial Doppler screening in the past 12 months, only count the patient once.</li> </ul>
Screening and Follow-up	SCDscreen3	<ul style="list-style-type: none"> <li>Proportion of parents/caregivers of newborns with a positive confirmatory test for SCD who received genetic education about SCD within 2 months of diagnosis</li> </ul>	<p><b>Numerator:</b> Count of parents/caregivers of newborns with a positive confirmatory test for SCD 2 months prior to the measurement month who received genetic education within 2 months of diagnosis (i.e., confirmatory test, second positive screen)</p> <p><b>Denominator:</b> Count of newborns with positive confirmatory test for SCD 2 months prior to the measurement month</p> <p><b>NOTE:</b></p> <ul style="list-style-type: none"> <li>Confirmatory test hemoglobin electrophoresis, not solubility test (sickle dex)</li> <li>For premature infants, clinicians should use best clinical judgment about date of initial screening,</li> <li>For premature infants who were transfused in the NICU repeat screen should be sent at least 4 months post transfusion</li> </ul>
	SCDscreen4	<ul style="list-style-type: none"> <li>Proportion of newborns with positive confirmatory test who completed follow-up appointment with hematologist within 90 days of diagnosis.</li> </ul>	<p><b>Numerator:</b> Count of newborns with a positive confirmatory screen for SCD in the month 3 months prior to the measurement month with hematology visit within 90 days of date of diagnosis (i.e., confirmatory test, second positive screen)</p> <p><b>Denominator:</b> Count of infants with positive confirmatory test in the month 3 months prior to the measurement month</p> <p><b>NOTE:</b></p> <ul style="list-style-type: none"> <li>Sickle cell specialists include hematologist, nurse practitioner or physician assistant specializing in sickle cell care</li> <li>The hematology or specialist visit must have been completed - referral alone is not sufficient for inclusion</li> </ul>
	SCDscreen17	<ul style="list-style-type: none"> <li>Percent of infants with initial screen positive for Sickle Cell Disease (FS) who had prophylactic antibiotics initiated by 90 days of diagnosis.</li> </ul>	<p><b>Numerator:</b> Count of infants with positive confirmatory test for Sickle Cell Disease (FS) who were born 3 months prior to the measurement month and who began prophylactic antibiotics within 90 days of diagnosis (i.e., confirmatory test, second positive screen)</p> <p><b>Denominator:</b> Count of infants with positive screen for Sickle Cell Disease (FS) who were born 3 months prior to the measurement month</p> <p><b>NOTE:</b></p> <ul style="list-style-type: none"> <li>“who began prophylactic antibiotics” refers to patients who received a prescription for penicillin. No confirmation of actual ingestion of penicillin is required.</li> </ul>
	SCDscreen19	<ul style="list-style-type: none"> <li>Percent of children with SCD-SS and SCD- SBeta zero thalassemia younger than 5 years who have a current prescription for prophylactic antibiotics.</li> </ul>	<p><b>Numerator:</b> Count of children with SCD-SS and SCD- SBeta zero thalassemia less than 5 years old with a current prescription for prophylactic antibiotics</p> <p><b>Denominator:</b> Count of children less than 5 years old with SCD-SS and SCD- SBeta zero thalassemia</p>

# INDIVIDUAL UTILIZATION DATA FORM

# SICKLE CELL DISEASE TREATMENT DEMONSTRATION PROGRAM INDIVIDUAL UTILIZATION QUESTIONNAIRE

Public Burden Statement: An agency may not conduct or sponsor, and a person is not required to respond to, a collection of information unless it displays a currently valid OMB control number. The OMB control number for this project is 0915-0344. Public reporting burden for this collection of information is estimated to average 45 minutes per response, including the time for reviewing instructions, searching existing data sources, and completing and reviewing the collection of information. Send comments regarding this burden estimate or any other aspect of this collection of information, including suggestions for reducing this burden, to HRSA Reports Clearance Officer, 5600 Fishers Lane, Room 10-29, Rockville, Maryland, 20857.

Subject ID Label: _____		Site: _____	
Today's Date:  __ _  -  __ _  - 20  __ _		Date Client Enrolled:  __ _  -  __ _  - 20  __ _	
Data Collector: _____			
Interview: 1 <input type="checkbox"/> Baseline 2 <input type="checkbox"/> Follow-up		Respondent: 1 <input type="checkbox"/> Sickle Cell Client: 2 <input type="checkbox"/> Other 3 <input type="checkbox"/> Both	

For each question, please indicate whether the information was obtained from (1) self-report by the Sickle Cell client or his/her proxy (e.g., caregiver), (2) a client data base, and/or (3) the client's medical records.

Baseline Interview Only. For FOLLOW-UP: BEGIN WITH QUESTION 5	
1. Age of client at time of interview: _____ years _____ months	Q.1 → <div style="border: 1px solid black; padding: 2px;">1. Self report 2. Database 3. Medical record</div>
2. Are you/Is the Client: 1 <input type="checkbox"/> Male 2 <input type="checkbox"/> Female	
3. What is (your/the client's) ethnic background? 1 <input type="checkbox"/> Hispanic 2 <input type="checkbox"/> Non-Hispanic	Q.2 → <div style="border: 1px solid black; padding: 2px;">1. Self report</div>
4. What is (your/the client's) race? (MARK ALL THAT APPLY) 1 <input type="checkbox"/> Black/African American 2 <input type="checkbox"/> White 3 <input type="checkbox"/> Native Hawaiian or Other Pacific Islander 4 <input type="checkbox"/> Asian 5 <input type="checkbox"/> American Indian or Alaskan Native	Q.3 → <div style="border: 1px solid black; padding: 2px;">1. Self report</div>  Q.4 → <div style="border: 1px solid black; padding: 2px;">1. Self report</div>

5. Including (yourself /the client), how many people live in the household?

\_\_\_\_|\_\_\_\_|

Q.5 →

- 1. Self report
- 2. Database
- 3. Medical record

6. What is the highest grade of school that (you/the client) completed?

- 0  Not school age
- 1  Currently in Grade School
- 2  Currently in Middle School
- 3  Currently in High School
- 4  Less than High School Graduate or GED
- 5  High School Graduate or GED
- 6  Post-High School Training other than College (Vocational, Technical, etc)
- 7  Some College
- 8  Graduated from College
- 9  Post-Graduate

Q.6 →

- 1. Self report
- 2. Database
- 3. Medical record

7. What type(s) of medical insurance (do you/does the client) have?  
(CHECK ALL THAT APPLY)

- 1  Medicaid 50 Medicare HMO
- 2  State Children's Health Insurance Plan (SCHIP)
- 3  Medicaid HMO
- 4  Medicare
- 5  Medicare HMO
- 6  Private
- 7  No Insurance
- 8  DON'T KNOW

Q.7 →

- 1. Self report
- 2. Database
- 3. Medical record

7a. Specify: \_\_\_\_\_

8. Please use this card (GIVE INCOME CARD) and tell me the number 1 through 11 that best represents your household yearly income from January 1st through December 31st of last calendar year, (SAY APPROPRIATE YEAR). Please include all sources of income.

Q.8 →

- 1. Self report
- 2. Database
- 3. Medical record

- |  |   |
|--|---|
| 1 <input type="checkbox"/> Less than \$5,000   | 8 <input type="checkbox"/> \$50,000 - \$59,999  |
| 2 <input type="checkbox"/> \$5,000 - \$9,999   | 9 <input type="checkbox"/> \$60,000 - \$79,999  |
| 3 <input type="checkbox"/> \$10,000 - \$14,999 | 10 <input type="checkbox"/> \$80,000 - \$94,999 |
| 4 <input type="checkbox"/> \$15,000 - \$19,999 | 11 <input type="checkbox"/> \$95,000 and over   |
| 5 <input type="checkbox"/> \$20,000 - \$29,999 | -8 <input type="checkbox"/> DON'T KNOW          |
| 6 <input type="checkbox"/> \$30,000 - \$39,999 | -9 <input type="checkbox"/> REFUSED             |
| 7 <input type="checkbox"/> \$40,000 - \$49,999 |   |

9. What type of Sickle Cell Disease (do you/does the client) have? (COLLECT SELF-REPORT RESPONSE AND VERIFY WITH DATABASE OR MEDICAL RECORD)

- |                                  | a. Self-Report              | b. Database/Medical Record  |
|----------------------------------|-----------------------------|-----------------------------|
| Sickle Cell Disease (SS)         | 1 <input type="checkbox"/>  | 1 <input type="checkbox"/>  |
| Sickle-Hemoglobin C Disease (SC) | 2 <input type="checkbox"/>  | 2 <input type="checkbox"/>  |
| Sickle Beta-Plus Thalassemia     | 3 <input type="checkbox"/>  | 3 <input type="checkbox"/>  |
| Sickle Beta-Zero Thalassemia     | 4 <input type="checkbox"/>  | 4 <input type="checkbox"/>  |
| Other: 9c. Specify: _____        | 5 <input type="checkbox"/>  | 5 <input type="checkbox"/>  |
| Dont Know                        | -8 <input type="checkbox"/> | -8 <input type="checkbox"/> |

10. At what age did (you/the client) first find out that (you have/the client has) Sickle Cell Disease?

- 1  NEWBORN SCREENING 2  OTHER: 10a. Specify Age: |\_\_|\_\_| year(s) old  
-8  DON'T KNOW  
-9  REFUSED

Q.10 →

1. Self report  
2. Database  
3. Medical record

We are interested in the health care that you receive from a variety of sources. These next questions ask about visits to a primary health care provider, a sickle cell specialist, other medical specialists, and a hospital emergency department.

11. In the past 12 months, how many times (have you/has the client) gone to a primary health care provider for:

- a. Sickle cell-related problems? |\_\_|\_\_|  
b. Non Sickle cell-related problems? |\_\_|\_\_|

Q.11a, b →

1. Self report  
2. Database  
3. Medical record

11c. Is (your/client's) primary health care provider also (your/his/her) sickle cell specialist?

- 1  Yes → SKIP TO Q.13 2  NO

12. In the past 12 months, how many times (have you/has the client) gone to a sickle-cell specialist (if not your primary care physician) for:

- a. Sickle cell-related problems? |\_\_|\_\_|  
b. Non Sickle cell-related problems? |\_\_|\_\_|

Qs.12a, b →

1. Self report  
2. Database  
3. Medical record

13. In the past 12 months, how many times (have you/has the client) gone to another type of specialist for:

- a. Sickle cell-related problems? |\_\_|\_\_|  
b. Non Sickle cell-related problems? |\_\_|\_\_|

Qs.13a, b →

1. Self report  
2. Database  
3. Medical record

14. In the past 12 months, did (you/the client) receive a referral for an eye examination?

- 1  Yes 2  No

Q.14 →

1. Self report  
2. Database  
3. Medical record

15. In the past 12 months, did (you /the client) make an appointment for an eye examination?

- 1  Yes → SKIP TO Q.16 2  No

Q.15a →

1. Self report  
2. Database  
3. Medical record

15a. Why wasn't an appointment made for an eye examination?

---

---

---

SKIP TO Q.17

16. Did (you/the client) go to the eye appointment?

1  Yes → SKIP TO Q.17    2  No

16a. Why didn't you (the client) go to the appointment?

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

17. In the past 12 months, how many times did (you/the client) receive health care services at a hospital emergency department

|\_|\_|\_|

Qs. 17a →  
1. Self report  
2. Database  
3. Medical record

18. In the past 12 months, (were you/was the client) admitted to the hospital?

1  Yes    2  No → SKIP TO Q.19

For each hospitalization, please tell me the number of nights and the reason (you were/the client was) in the hospital. (LIST ADDITIONAL STAYS ON BACK OF PAGE)

18a. Hospital Stay	18b. # of nights	18c. Reason
#1	_ _ _	_____
#2	_ _ _	_____
#3	_ _ _	_____
#4	_ _ _	_____
#5	_ _ _	_____

Qs. 18a-c →  
1. Self report  
2. Database  
3. Medical record

19. (Are you/is the client) currently taking hydroxyurea therapy?

1  Yes → SKIP TO Q.21    2  No

20. In the past 12 months has (your/client's) physician discussed hydroxyurea therapy as an option for (you/the client)?

1  Yes    2  No

Q. 19 →  
1. Self report  
2. Database  
3. Medical record

21. What is (your/client's) baseline hemoglobin level? (COLLECT SELF-REPORT RESPONSE AND VERIFY WITH DATABASE OR MEDICAL RECORD).

a. Self-Report

|\_|\_|\_|.|\_|

-8  DON'T KNOW

b. Database/Medical Record

|\_|\_|\_|.|\_|

-9  NO ACCESS TO DATABASE/MEDICAL RECORD

Q. 20 →  
1. Self report  
2. Database  
3. Medical record



22. **BASELINE:** (Have you/Has the client) ever had the following Sickle Cell complications?  
**FOLLOW-UP:** In the past 12 months, (have you/has the client) had the following Sickle Cell Complications?

		Yes	No	Don't Know	N/a
a.	Pain	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
b.	Sickling in the lungs	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
c.	Fever	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
d.	Severe infection	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
e.	Stroke	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
f.	Kidney damage	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
g.	Leg ulcers	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
h.	Sickle eye damage	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
i.	Gall bladder attack	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
j.	Priapism	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
k.	Hand-foot syndrome	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
l.	Spleen problems	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
m.	Seizures	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	
n.	Other	1 <input type="checkbox"/> ↓	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	

Qs. 22a-n →

- 1. Self report
- 2. Database
- 3. Medical record

Please Specify: \_\_\_\_\_

Qs. 23 →

- 1. Self report
- 2. Database
- 3. Medical record

23. **BASELINE:** (Have you/has the client) ever been given regularly scheduled blood transfusions?  
**FOLLOW-UP:** In the past 12 months, (have you/has the client) been given regularly scheduled blood transfusions?  
 1  Yes 20  No

24. **BASELINE:** (Have you/has the client) ever been counseled on the following?  
**FOLLOW-UP:** In the past 12 months, (have you/has the client) been counseled on the following?

		Yes	No	Don't Know
a.	SCD complications	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>
b.	Inheritance of SCD	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>

Qs. 24a,b →

- 1. Self report
- 2. Database
- 3. Medical record

**IF CLIENT IS 6 YEARS OR OLDER, SKIP TO Q. 27**

25. **Is the client taking prophylactic antibiotics (i.e., penicillin)?**

1  Yes → SKIP TO Q.26 2  No

Qs. 25a →

- 1. Self report
- 2. Database
- 3. Medical record

**25a. Why isn't the client taking prophylactic antibiotics?**

\_\_\_\_\_

\_\_\_\_\_

**SKIPTO Q.27**

26. **At what age did the client start taking prophylactic antibiotics?**

\_\_\_\_|\_\_\_\_| 1  weeks 3  years 2  months -8  Don't know

Qs. 26a →

- 1. Self report
- 2. Database
- 3. Medical record

**26a. How often is the client taking prophylactic antibiotics?**

- 1  2 times per day
- 2  1 time per day
- 3  Less than 1 time per day

Qs. 27a-k →

- 1. Vacc. card
- 2. Database
- 3. Medical record

**27. (Have you/Has the client) had:**

**For children only:**

	Yes	No	Don't Know	N/a
a. Developmental screening to monitor infant's/ child development in areas of communication, motor, social, problem-solving and self-help skills?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>

**For all participants:**

b. A dental exam in the last year?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
c. Hearing screening in the last year?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
d. Vision screening in the last year?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
e. Diabetes screening in the last year?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
f. Blood pressure check in the last year?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
g. TCD (Transcranial Doppler) in the last year?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>

**For adults only:**

h. A mammogram in the in last 2 years?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
i. A pap smear in the last 3 years?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
j. Colon screening in the last 10 years?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
k. A PSA Test?	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>

**THE FOLLOWING INFORMATION SHOULD BE OBTAINED ONLY FROM A VACCINATION CHART, CLIENT DATA BASE OR CLIENT MEDICAL RECORD.**

**FOR CLIENTS AGED 6 YEARS AND YOUNGER**

Q. 28a →

- 1. Vacc. Card
- 2. Database
- 3. Medical record

**28a. INDICATE WHETHER OR NOT THE CLIENT IS UP-TO-DATE WITH THE FOLLOWING VACCINATIONS:**

	Yes	No	Don't Know	N/a
(1) Diphtheria, Tetanus, Pertussis (DTaP)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(2) Meningococcal (MCV4 or MPSV4)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(3) Pneumococcal Conjugate Vaccine	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(4) Pneumococcal Polysaccharide Vaccine	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(5) Influenza	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(6) Hepatitis A (Hep A)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(7) Hepatitis B (Hep B)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(8) Inactivated Poliovirus (IPV)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(9) Measles, Mumps, Rubella (MMR)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(10) Varicella	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(11) Rotavirus (Rotateq)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(12) Haemophilus influenza type b (Hib)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>

**FOR CLIENTS AGED 7 TO 18 YEARS**

Q. 28b →

- 1. Vacc. Card
- 2. Database
- 3. Medical record

**28b. INDICATE WHETHER OR NOT THE CLIENT IS UP-TO-DATE WITH THE FOLLOWING VACCINATIONS:**

		Yes	No	Don't Know	N/a
(1)	Diphtheria, Tetanus, Pertussis (Tdap)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(2)	Meningococcal (MCV4 or MPSV4)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(3)	Pneumococcal Polysaccharide Vaccine	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(4)	Influenza	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(5)	Hepatitis A (Hep A)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(6)	Hepatitis B (Hep B)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(7)	Inactivated Poliovirus (IPV)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(8)	Measles, Mumps, Rubella (MMR)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(9)	Varicella	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(10)	Human Papillomavirus (HPV)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>

**FOR CLIENTS AGED 19 YEARS AND OLDER**

Q. 28c →

- 1. Vacc. Card
- 2. Database
- 3. Medical record

**28c. INDICATE WHETHER OR NOT THE CLIENT IS UP-TO-DATE WITH THE FOLLOWING VACCINATIONS:**

		Yes	No	Don't Know	N/a
(1)	Diphtheria, Tetanus, Pertussis (Td/Tdap)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(2)	Meningococcal (MCV4 or MPSV4)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(3)	Pneumococcal Polysaccharide Vaccine	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(4)	Influenza	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(5)	Hepatitis A (Hep A)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(6)	Hepatitis B (Hep B)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(7)	Measles, Mumps, Rubella (MMR)	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(8)	Varicella	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(9)	Human Papillomavirus	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>
(10)	Zoster	1 <input type="checkbox"/>	2 <input type="checkbox"/>	-8 <input type="checkbox"/>	-7 <input type="checkbox"/>

# PEDIATRIC QUALITY OF LIFE INVENTORY

# PEDSQL™ PEDIATRIC QUALITY OF LIFE INVENTORY

Version 4.0 Short Form (SF15)  
TEEN REPORT (ages 13-18)

ID#: \_\_\_\_\_  
Date: \_\_\_\_\_

OMB Number: 0915-0344  
Expiration Date: 12/31/2014

Public Burden Statement: An agency may not conduct or sponsor, and a person is not required to respond to, a collection of information unless it displays a currently valid OMB control number. The OMB control number for this project is 0915-0344. Public reporting burden for this collection of information is estimated to average 12 minutes per response, including the time for reviewing instructions, searching existing data sources, and completing and reviewing the collection of information. Send comments regarding this burden estimate or any other aspect of this collection of information, including suggestions for reducing this burden, to HRSA Reports Clearance Officer, 5600 Fishers Lane, Room 10-29, Rockville, Maryland, 20857.

## DIRECTIONS

On the following page is a list of things that might be a problem for you. Please tell us **how much of a problem** each one has been for you during the **past ONE** month by circling:

- 0 if it is **never** a problem
- 1 if it is **almost never** a problem
- 2 if it is **sometimes** a problem
- 3 if it is **often** a problem
- 4 if it is **almost always** a problem

There are no right or wrong answers.  
If you do not understand a question, please ask for help.

In the past **ONE** month, how much of a **problem** has this been for you...

ABOUT MY HEALTH AND ACTIVITIES (problems with...)	Never	Almost Never	Sometimes	Often	Almost Always
1. It is hard for me to walk more than one block	0	1	2	3	4
2. It is hard for me to run	0	1	2	3	4
3. It is hard for me to do sports activity or exercise	0	1	2	3	4
4. It is hard for me to lift something heavy	0	1	2	3	4
5. It is hard for me to do chores around the house	0	1	2	3	4

ABOUT MY FEELINGS (problems with...)	Never	Almost Never	Sometimes	Often	Almost Always
1. I feel afraid or scared	0	1	2	3	4
2. I feel sad or blue	0	1	2	3	4
3. I feel angry	0	1	2	3	4
4. I worry about what is going to happen to me	0	1	2	3	4

HOW I GET ALONG WITH OTHERS (problems with...)	Never	Almost Never	Sometimes	Often	Almost Always
1. I have trouble getting along with other teens	0	1	2	3	4
2. Other teens do not want to be my friend	0	1	2	3	4
3. Other teens tease me	0	1	2	3	4

ABOUT SCHOOL (problems with...)	Never	Almost Never	Sometimes	Often	Almost Always
1. It is hard for to pay attention in class	0	1	2	3	4
2. I forget things	0	1	2	3	4
3. I have trouble keeping up with my schoolwork	0	1	2	3	4

## SF-8 HEALTH SURVEY

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## YOUR HEALTH AND WELL BEING

*This survey asks for your views about your health. This information will help keep track of how you feel and how well you are able to do your usual activities.*

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1. **Overall, how would you rate your health during the past 4 weeks?  
(Check the one box that best describes your answer.)**

Excellent      Very good      Good      Fair      Poor      Very Poor  
 1       2       3       4       5       6

2. **During the past 4 weeks, how much did physical health problems limit your usual physical activities (walking, climbing stairs)?**

Not at all      Very little      Somewhat      Quite a lot      Could not do physical activities  
 1       2       3       4       5

3. **During the past 4 weeks, how much difficulty did you have doing your daily work, both at home and away from home, because of your physical health?**

Not at all      A little bit      Some      Quite a lot      Could not do daily work  
 1       2       3       4       5

4. **How much bodily pain have you had during the past 4 weeks?**

None      Very mild      Mild      Moderate      Severe      Very Severe  
 1       2       3       4       5       6

5. **During the past 4 weeks, how much energy did you have?**

Very much      Quite a lot      Some      A little      None  
 1       2       3       4       5



6. During the past 4 weeks, how much did your physical health or emotional problems limit your usual social activities with family or friends?

Not at all      Very little      Somewhat      Quite a lot      Could not do social activities  
■ 1      ■ 2      ■ 3      ■ 4      ■ 5

7. During the past 4 weeks, how much have you been bothered by emotional problems (such as feeling anxious, depressed or irritable)?

Not at all      Slightly      Moderately      Quite a lot      Could not do daily activities  
■ 1      ■ 2      ■ 3      ■ 4      ■ 5

8. During the past 4 weeks, how much did personal or emotional problems keep you from doing your usual work, school or other daily activities?

Not at all      Very little      Somewhat      Quite a lot      Could not do daily activities      Very Severe  
■ 1      ■ 2      ■ 3      ■ 4      ■ 5      ■ 6

## CLIENT SURVEY DATA, TABLES, AND FIGURES

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**TABLE 1:** Individual utilization surveys by participating sites

Site	Baseline	Follow-up 1	Follow-up 2	Follow-up 3	Total Surveys
CA	115	86	32	0	233
CO	63	35	1	0	99
IL	247	139	6	0	392
MD	118	73	2	0	193
MO	79	17	0	0	96
NJ	116	63	1	0	180
OH	107	80	36	21	244
PA	95	50	2	0	147
TN	100	59	0	0	159
<b>TOTAL</b>	<b>1040</b>	<b>602</b>	<b>80</b>	<b>21</b>	<b>1743</b>

**TABLE 2:** Description of patients enrolled in SCDTDP (N=1040)

Characteristic	Study Population, No. (%)
Total N	1040
<b>Age, y</b>	
Mean	24.3 (15.1)
Median	22.2 (0-79)
<b>Gender</b>	
Female	59.60%
Male	40.40%
<b>Race</b>	
Black/African American	979 (94.1)
White	14 (1.4)
Native Hawaiian or other Pacific Islander	5 (0.5)
Asian	2 (0.2)
American Indian or Alaskan Native	32 (3.1)
<b>Ethnicity</b>	
Hispanic	2.50%
<b>Number of members in household</b>	
Mean	3.6 (1.9)
Median	3 (1-5)

**TABLE 3:** Sickle cell disease type at baseline

Sickle Cell Disease Type	Self Report (n=882), %	Medical Record (n=775), %
Sickle Cell Disease (SS)	71.9	60.7
Sickle Hemoglobin C Disease (SC)	15.5	9.8
Sickle Beta-Plus Thalassemia	4.1	4.4
Sickle Beta-Zero Thalassemia	0.9	1.6
Other	1.0	8.0
Don't Know	6.6	15.6

Average number of visits to primary health care provider and sickle cell disease specialist at baseline and follow up (12 month period before the second visit)

**TABLE 4:**

Visit to Primary Health Care Provider	Sickle cell-related Mean (SD) Median (range) p = 0.5210	Non Sickle cell-related Mean (SD) Median (range) p= 0.1985
Baseline	3.5 (5.5) 1 (0-48)	2.1 (3.1) 1 (0-30)
Follow-up I	3.6 (5.8) 1 (0-50)	1.9 (2.7) 1 (0-30)

Visit to Sickle Cell Specialist	Sickle cell-related Mean (SD) Median (range) p =0.8850	Non Sickle cell-related Mean (SD) Median (range) p =0.9149
Baseline	5.8 (8.2) 4 (0-104)	0.6 (1.8) 0 (0-20)
Follow-up I	5.8 (7.0) 4 (0-75)	0.7 (2.0) 0 (0-20)

**TABLE 5:** Hospitalizations, emergency department visits, treatment and counseling

Percent Admitted to Hospital in Past 12 Months and Average Number of Hospital Stays		
	Hospital Admissions** n (%) p=0.0044	Average Number of Hospital Stays Mean (SD) p=0.3706
Baseline	649/1017 (63.8)	2.3 (1.6)
Follow Up	330/583 (56.6)	2.4 (1.6)

Average Number of visits to ER in the Past 12 Months		
	Mean (SD) Median (range)	
Baseline (n=1016)	4.0 (7.1) 2 (0-100)	
Follow-up I (n=586)	3.5 (5.7) 2 (0-50)	

**TABLE 5:** Cont.

Currently Taking Hydroxyurea & Counseled by Physician about Hydroxyurea in the Past 12 Months		
	Taking Hydroxyurea N=1605 (%) p=0.2507	Counseled about Hydroxyurea** N=954 (%) p=0.6623
Baseline	389/1017 (38.3)	254/618 (41.1)
Follow-up I	242/588 (41.2)	143/336 (42.6)

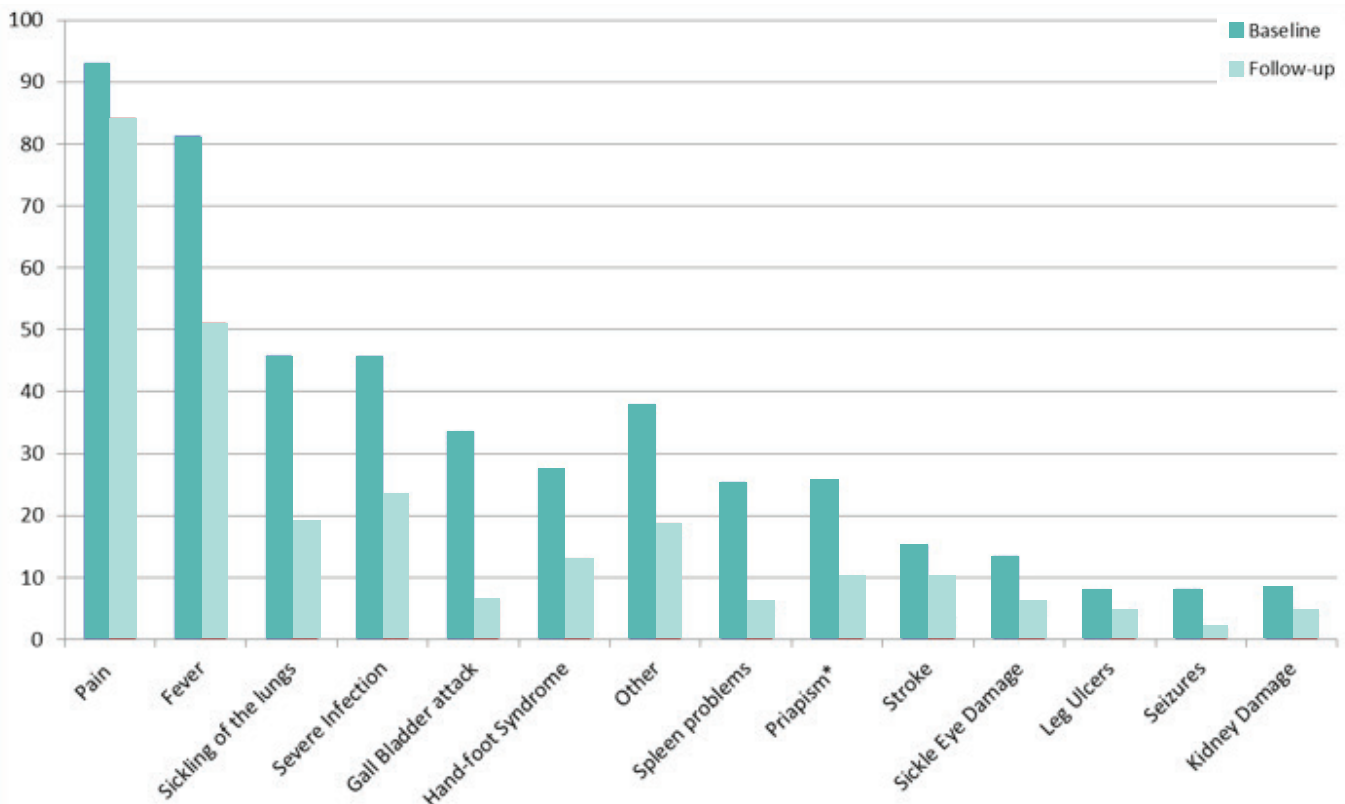
**TABLE 6:** Regularly scheduled transfusions in the past 12 months

Regular Blood Transfusions in the past 12 months** n=992 P<0.0001		
Baseline (n=420)	168 (40.0%)	
Follow-up (n=572)	153 (26.7%)	

**TABLE 7:** Prevalence of antibiotic use in children

Age Group	Baseline, (%)	Follow Up, (%)
< 5 yrs	59/61 (96.7%)	24/25 (96%)

**FIGURE 1:** Figure 1. Complications due to sickle cell disease at baseline and follow-up



Note: The question asked was: In the past 12 months, (have you/has the client) had the following sickle cell complications?

**TABLE 8:** Complications from sickle cell disease\*\*

Type	N	Baseline %	N	Follow-up %	P-value
Pain	1024	93.1	592	84.12	<0.0001
Fever	1021	81.3	590	51.0	<0.0001
Sickling of the lungs	1019	45.9	591	18.8	<0.0001
Severe infection	1024	45.7	591	23.2	<0.0001
Gall bladder attack	1018	33.6	592	6.4	<0.0001
Hand-foot Syndrome	1012	27.6	588	12.9	<0.0001
Other	912	37.8	543	18.6	<0.0001
Spleen problems	1018	25.3	590	5.9	<0.0001
Priapism*	396	24.8	30	10.0	0.1588
Stroke	1019	15.3	592	2.2	<0.0001
Sickle Eye Damage	1017	13.5	592	5.9	<0.0001
Leg Ulcers	1018	7.9	589	4.4	<0.0071
Seizures	1019	8.1	587	1.9	<0.0001
Kidney Damage	1017	8.3	592	4.6	0.0039

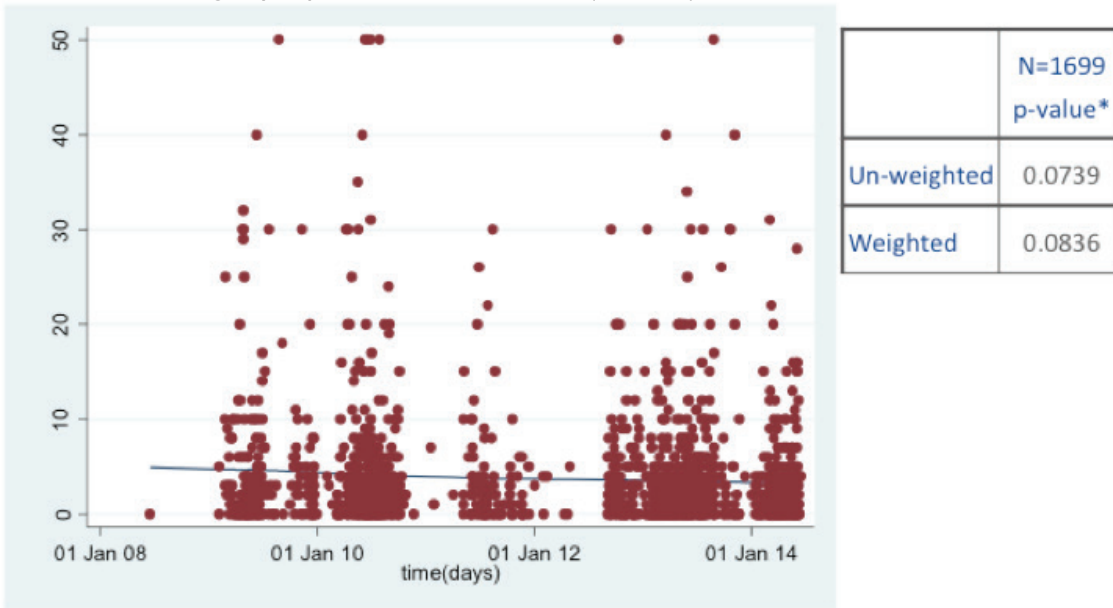
**TABLE 9:** Counseling for sickle cell disease complications/inheritance of sickle cell disease

	Counseling for SCD Complications N (%) P<0.0001	Counseling for Inheritance of SCD N (%) P<0.0001
Baseline	771 (75.4%)	788 (77.1%)
Follow-up	363 (61.8%)	293 (50.1%)

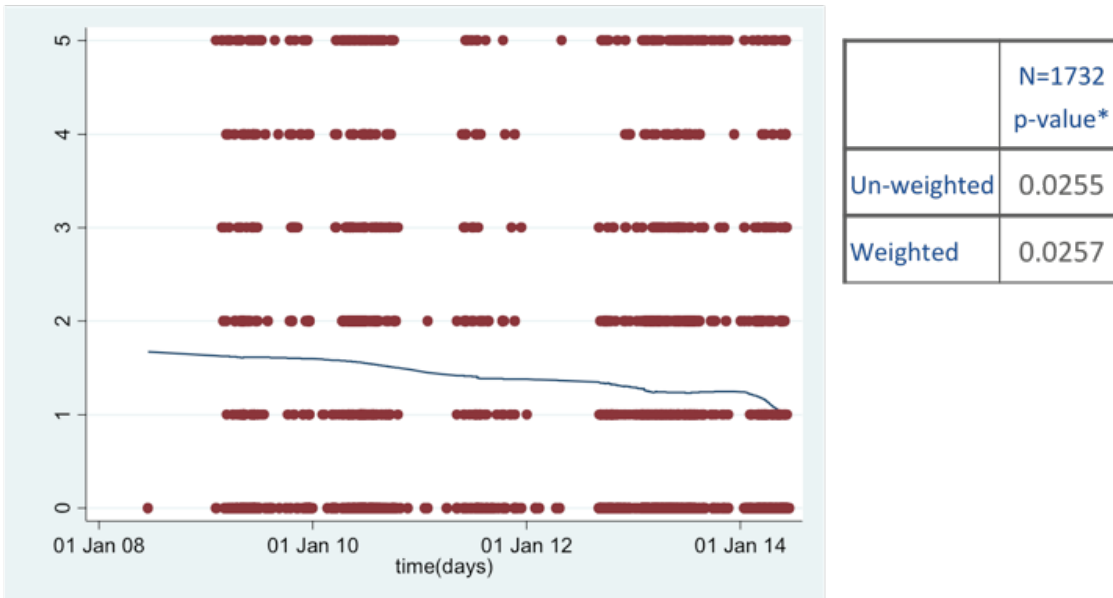
**TABLE 10:** Routine preventative screening for patients with sickle cell disease

Type	Baseline %		Follow-up		P-value
	N	Percent	N	Percent	
Colon (age ≥50)	68	52.9	31	67.7	0.0939±
Blood Pressure	1015	94.1	588	94.4	0.4432±
Vision	1012	60.1	588	62.2	0.7300±
Dental	1010	59.9	590	61.2	0.7424±
Pap Smear (Female age>15)	455	62.2	44	52.3	0.0029
Hearing	1009	36.8	589	38.0	0.7350
Transcranial Doppler (ages 2-16)	322	52.2	137	46.0	0.6071
Developmental (age≤18)	332	37.4	147	25.2	<0.0001
Prostate [PSA] (Male age ≥50)	27	55.6	1	0	--
Mammogram (Female age≥40)	95	63.2	8	50.0	0.4733±
Diabetes	1008	21.9	583	27.8	0.0130

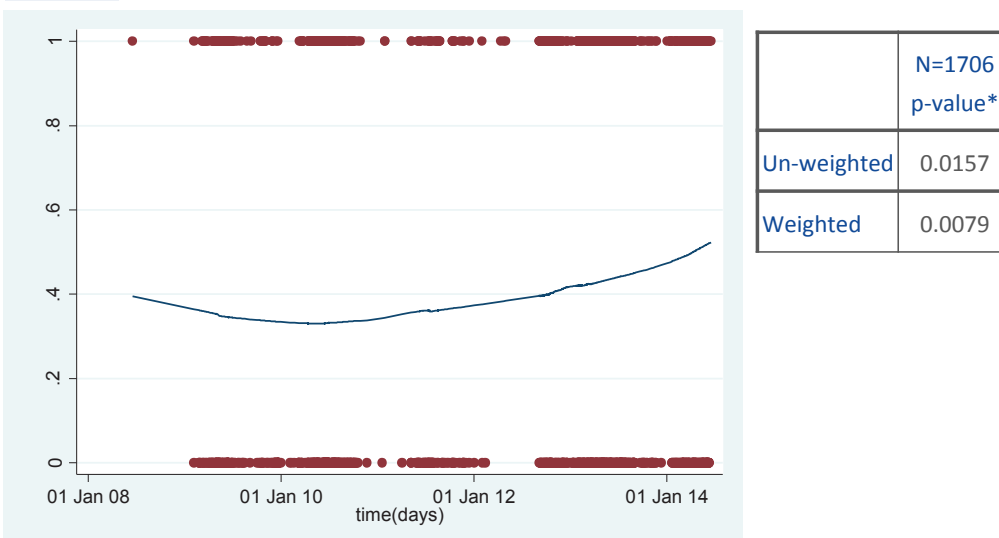
**FIGURE 2:** Emergency department visits over time (all teams)



**FIGURE 3:** Frequency of hospitalizations



**FIGURE 4:** Figure 4: Hydroxyurea use over time



**TABLE 11:** PedsQLTM survey by sites

Site	Baseline	Follow-up 1	Follow-up 2	Follow-up 3	Total Surveys
CA	37	32	18	5	92
CO	25	13	0	0	38
IL	98	55	3	3	159
MD	15	9	0	0	24
MO	24	10	0	0	34
NJ	18	8	0	0	26
PA	35	21	0	0	56
TN	78	56	0	0	134
<b>TOTAL</b>	<b>330</b>	<b>204</b>	<b>21</b>	<b>8</b>	<b>563</b>

**TABLE 12:** PedsQLTM domains at baseline, follow up, and overall

Domain	Baseline		Follow-up 2		Overall		p-value
	N	Mean (SD)	N	Mean (SD)	N	Mean (SD)	
Total PedsQL	314	73.2 (17.3)	130	74.7 (17.3)	444	73.7 (17.3)	0.4041
Physical Functioning	314	74.5 (22.4)	130	74.3 (23.4)	444	74.4 (22.7)	0.9196
Emotional Functioning	314	73.1 (19.8)	130	74.8 (20.3)	444	73.6 (19.9)	0.4284
Social Functioning	302	78.4 (21.7)	130	82.3 (19.9)	432	79.6 (21.2)	0.0803
School Functioning	293	65.9 (24.7)	124	67.8 (23.8)	417	66.4 (24.5)	0.4489

**TABLE 13:** Parent PedsQLTM 4.0 domains comparison of collaborative to literature by mean and median

Domain <sup>^</sup>	N	NICHQ Collaborative* Mean (SD)	N	Literature 1** Mean (SD)	N	Literature 2 ‡ Mean (n, SD)
Total PedsQL	444	73.7 (17.3)	1769	71.27 (18.43)	123	62.9 (17.7)
Physical Functioning	444	74.4 (22.7)	1769	71.13 (22.97)	123	60.4 (22.2)
Emotional Functioning	444	73.6 (19.9)	1756	73.06 (20.22)	122	66.6 (21.64)
Social Functioning	432	79.6 (21.2)	1763	77.44 (21.51)	123	68.4 (23.8)
School Functioning	417	66.4 (24.5)	1665	62.13 (23.26)	123	57.5 (20.1)

Domain <sup>^</sup>	N	NICHQ Collaborative* Median (IQR)	N	Literature 3** Children with SCD Median (IQR)	N	Literature 3** Children without SCD Median (IQR)
Total PedsQL	444	74.0 (26.9)	104	67.4 (50.0, 83.5)	74	80.7 (58.7, 92.4)
Physical Functioning	444	75.0 (40.0)	104	68.8 (50.0, 87.5)	74	87.5 (56.3, 96.9)
Emotional Functioning	444	75.0 (34.9)	104	72.5 (60.0, 90.0)	74	75.0 (55.0, 90.0)
Social Functioning	432	83.3 (37.5)	104	75.0 (55.0, 90.0)	74	86.3 (60.0, 100.0)
School Functioning	417	66.7 (37.5)	97	55.0 (40.0, 70.0)	69	66.7 (50.0, 90.0)

NICHQ Collaborative score is the overall mean from REDCap data

\*\* Scores from Comprehensive Sickle Cell Centers (CSCC) Clinical Trial Consortium (CTC); Dampier et. al (2010)

‡ Scores from Health-related Quality of Life in Children and Adolescents With Sickle Cell Disease ;

Dale JC et. al (2011)

<sup>^</sup> PedsQL scores are from parent surveys only



**TABLE 14:** SF Health Survey by sites

Site	Baseline	Follow-up 1	Follow-up 2	Total Surveys
CA	81	60	24	165
CO	39	21	1	61
IL	107	18	0	125
MD	103	62	0	165
MO	49	3	0	52
NJ	98	51	0	149
OH	87	43	21	151
PA	61	28	0	89
TN	14	2	0	16
<b>TOTAL</b>	<b>639</b>	<b>288</b>	<b>46</b>	<b>973</b>

**TABLE 15:** SF Health Survey domains at baseline, follow up, and overall

Domain	Baseline		Follow-up 2		Overall		p-value
	N	Mean (SD)	N	Mean (SD)	N	Mean (SD)	
Total SF Score	638	59.5 (20.2)	283	60.0 (21.0)	921	59.7 (20.4)	0.7202
Physical Functioning	637	62.0 (26.6)	283	62.5 (26.4)	920	62.2 (26.5)	0.7910
Emotional Functioning	447	63.7 (22.3)	246	63.2 (23.5)	693	63.5 (22.7)	0.7443
Social Functioning	633	65.2 (27.7)	280	66.1 (28.9)	913	65.5 (28.1)	0.6607
Pain	635	48.2 (26.9)	280	47.7 (27.8)	915	48.1 (27.2)	0.7815
General Health	637	51.0 (22.9)	282	52.4 (23.8)	919	51.4 (23.2)	0.3913

**TABLE 16:** Table 16. Demographics and outcome comparisons between prior and current National Coordinating Center (NCC)

			Prior NCC		Current NCC		P-value	Odds Ratio
			N		N			
Age		Mean (SD), Median (Range)	23.4 (14.6) 22 (0-77)		24.9 (15.1) 23.6 (0-80)		0.0284	
No. in household		Mean (SD), Median (Range)	3.5 (1.8) 3 (1-15)		3.6 (1.9) 3 (1-15)		0.1915	
Gender	Male	N (%)	430 42.2		387 33.1		< 0.0001	
	Female	N (%)	588 57.8		783 66.9			
Race/Ethnicity	Hispanic	N (%)	22 2.2		33 2.8		0.3403	
	Black/African American	N (%)	665 65.3		720 60		0.8800	
	White	N (%)	8 0.8		12 1			
	Native Hawaiian or other Pacific Islander	N (%)	3 0.3		4 0.3			
	Asian	N (%)	1 0.1		2 0.2			
	American Indian	N (%)	22 2.2		19 1.6			
Outcomes	ED visits	Mean (SD), Median (Range)	3.9 (7.2) 2 (0-67)		3.4 (6.0) 2 (0-100)		0.2494 <sup>^</sup> 0.3106 <sup>^^</sup>	
	# of Hospitalizations	Mean (SD), Median (Range)	1.3 (1.6) 1 (0-5)		1.3 (1.6) 1 (0-5)		0.2769 <sup>^</sup> 0.9001 <sup>^^</sup>	
	Hydroxyurea Use	N (%)	322 32.1		505 42.2		<0.0001	1.546
	Hydroxyurea Counseling	N (%)	234 34.8		309 45.6		<0.0001	1.567

