Sickle Cell Disease
Access-to-Care Summit
2018

Electronic version available at GBT.com.

Sponsored by Global Blood Therapeutics, Inc
# Table of Contents

2 Abbreviations

3 Introduction

6 Enhancing Care Within the Institution: Establishing a Sickle Cell Disease Clinic and/or Day Hospital for Comprehensive Care Management

11 Utilizing Existing Care Infrastructure: Leveraging Available Capacity by Partnering with Non-sickle cell disease Stakeholders

16 Transitioning Adolescent Care: Structuring Transition Between Pediatric and Adult Care Settings

21 Expanding Care Outside the Institution: Building a Community Outreach Model to Extend the Reach of Care

27 References

## Abbreviations

CBO=community-based organization  
DH=day hospital  
ECHO=Extension for Community Healthcare Outcomes  
ED=emergency department  
HCPs=healthcare providers  
NBS=newborn screening  
PCP=primary care provider  
SMART=Specific, Measurable, Achievable, Relevant, and Time-bound  
US=United States
“Of all the forms of inequality, injustice in health care is the most shocking and inhumane.”

- Dr. Martin Luther King, Jr.

Introduction

The Sickle Cell Disease Access-to-Care Summit, held on September 12, 2018 brought together committed stakeholders from across the United States to explore ways of improving the lives of people living with the disease who labor under a myriad of burdens, blockades and beliefs that have weighed upon African Americans for four centuries. Moreover, as a chronic illness sickle cell disease brings additional hardships and challenges that are unbounded by race, religion or national origin. In this context of institutional and systemic racism, people living with the disease face a system of parallel, fragmented care that rests on an unwieldy infrastructure and a healthcare environment riddled with injustice.

A Steering Committee of eight stakeholders from academia and business (Figure 1) formulated the program and set the objectives which were to:

- Identify and discuss existing access-to-care models used successfully to improve healthcare for both adults and children with sickle cell disease
- Create draft roadmaps of successful access-to-care models by involving stakeholders from across the healthcare spectrum
- Develop implementation roadmaps suitable for dissemination through existing channels to local and regional access-to-care initiatives

Figure 1. Steering Committee Members

Biree Andemariam, MD
Associate Professor, Department of Medicine
Director, New England Sickle Cell Institute and Connecticut Bleeding Disorders Center
University of Connecticut
Farmington, CT

Andrew Campbell, MD
Director, Comprehensive Sickle Cell Program
Children’s National Medical Center
Associate Professor of Pediatrics
George Washington University School of Medicine and Health Sciences
Division of Hematology
Washington, DC

Kim Smith-Whitley, MD
Director, Comprehensive Sickle Cell Center at Children’s Hospital of Philadelphia
Clinical Director, Division of Hematology
Associate Professor of Pediatrics
Perelman School of Medicine at the University of Pennsylvania
Philadelphia, PA

Payal Desai, MD
Director, Sickle Cell Program
Director, Sickle Cell Research
Assistant Professor of Internal Medicine
Division of Hematology
The Ohio State University
Columbus, OH

John J. Strouse, MD, PhD
Director of the Adult Sickle Cell Program
Associate Professor of Medicine and Pediatrics
Duke University School of Medicine
Durham, NC

Julie Kanter, MD
Director, Adult Sickle Cell Program
University of Alabama at Birmingham
Birmingham, Alabama

James R. Eckman, MD
Professor Emeritus, Hematology & Oncology
Emory University School of Medicine
Atlanta, Georgia

Kenneth Bridges, MD
Vice President, Medical Affairs and Principle Medical Director
Global Blood Therapeutics
South San Francisco, CA
Founder and Former Director, Joint Center for Sickle Cell and Thalassemic Disorders
Brigham & Women’s and Massachusetts General Hospitals
Boston, MA
Access-to-Care challenges were addressed under four overarching headings:

- Expanding care within the institution: establishing a sickle cell disease clinic and/or day hospital for comprehensive management
- Utilizing existing care infrastructure: leveraging available institutional capacity by partnering with stakeholders outside the sickle cell disease arena
- Transitioning adolescent care: structuring transition between pediatric and adult care facilities
- Expanding care outside the institution: building outreach models to extend the range of care into the community and beyond

Participating stakeholders included patient advocates, health care professionals (HCPs), hospital administrators, as well as representatives of professional medical societies and government healthcare agencies. The Steering Committee created draft roadmaps to kindle group discussions, the output of which is distilled into the guidance material below. The resulting implementation roadmaps are freely available to interested stakeholders for use and adaptation to individual circumstances.

The US healthcare system is a patchwork of programs and institutions built on a funding infrastructure that includes private, local, state, and federal sources. The focus of healthcare often is short-term with problems addressed most often on a year-to-year basis, an approach best suited to acute issues of limited duration. Acquiring adequate healthcare can be challenging for people who are adept within the system and well-resourced. For those who are poor and marginalized the problem is daunting. For poor, marginalized people with chronic illness the mountain is almost insurmountable.

Sickle cell disease is a complex, chronic illness affecting approximately 100,000 Americans (Figure 2). The multifaceted disorder evolves over decades in character and manifestations. Except for the limited option of bone marrow transplantation, no cure exists. The problems are further exacerbated by the fact that most physicians are unfamiliar and/or uncomfortable with sickle cell disease management. Episodes of acute vaso-occlusive pain are the hallmark of sickle cell disease and over the course of a year these drive most patients into an emergency department (ED) on one or more occasions. Unfortunately, the ED is an option of...
Sickle cell disease is a chronic disorder that results from a single amino acid substitution in the hemoglobin molecule. Clinical manifestations begin in infancy and extend into adulthood. The life-saving intervention of childhood prophylactic penicillin was the catalyst for sickle cell disease newborn screening programs which currently exist in each state. Efficient screening technology combined with effective treatment created a moral imperative to implement sustained and comprehensive care for children with sickle cell disease. Although the lifespan for patients with sickle cell disease lags the average for African Americans, survival has increased beyond that reported in the Cooperative Study of Sickle Cell Disease, with improved childhood survival contributing significantly to the better numbers.

Young adults generally transition from pediatric to adult healthcare systems between the ages of 18 and 25 years. Unfortunately, the dearth of benign hematology providers has created a crisis in the management of adults with sickle cell disease. The problem is exacerbated by infrastructure gaps common in the adult world. The integrated, multidisciplinary approach to disease management that is a bedrock in pediatrics sadly is a rarity for adults. Children and parents come to see the pediatric clinic as a haven that at times serves nearly as a second home. Young adults are understandably anxious when considering where they will receive treatment that avoids many of the shortcomings of ED management. A dedicated facility staffed by HCPs familiar both with the clinical manifestations of sickle cell disease and its treatment provides a stable setting for comprehensive management of a complex disorder. Intensive treatment of vaso-occlusive pain in DHs consistently reduces the rate of hospital admission for uncomplicated episodes relative to the ED. Moreover, DHs can evaluate uncomplicated vaso-occlusive pain crises and begin analgesics much more rapidly than occurs in the ED. Consequently, patient satisfaction is greater with DH treatment leading many patients to seek these facilities in lieu of the ED. In fact, patients often manage pain at home while waiting for DH facilities to open rather than seeking ED treatment. Importantly, reductions in hospitalizations and length of stay by DHs can substantially reduce the cost of care for patients with sickle cell disease. By channeling patients into an alternate management setting, DHs potentially can free ED facilities and personnel to focus on problems they handle best, such as major trauma or acute myocardial infarction.

Drs. Andemariam and Eckman summarize the implementation roadmap for establishing DHs and/or sickle cell disease treatment clinics. Starting such programs often involves tapping into other departments at an institution whose resources can be shared. Comprehensive Cancer Centers are an example where synergy possibly exists, particularly in the hematologic malignancy sections where staff are familiar with issues involving the blood-forming elements. Drs. Desai and Strouse address this approach to expanding access-to-care for people with sickle cell disease.
treatment in the adult world and by whom. Most patients endeavor to remain in the pediatric environment for as long as possible. Unfortunately, delay places patients at risk of having no transition at all.

The issue of pediatric to adult transition for patients with sickle cell disease is complex with multiple components that include the number of patients served and the proximity of adult and pediatric facilities. Drs. Campbell and Smith-Whitley address this central challenge in access to care. Importantly, even geographic location within the country can influence the outcome of transition. The road to adult care sadly has more potholes for patients in some regions of the country.

Geography plays another unfortunate and even broader negative role in access-to-care for people with sickle cell disease. In 2010, 55 percent of black Americans lived in the South, and 105 Southern counties had a black population of 50 percent or higher. Rural healthcare has traditionally lagged that available in urban areas. The picture has changed dramatically over the past decade due to a general transformation of health care financing, the introduction of new technologies, and the clustering of health services into systems and networks. Despite these changes, resources for rural health systems remain relatively insufficient. Many rural communities continue to experience shortages of physicians, and the proportion of rural hospitals under financial stress is much greater than that of urban hospitals.

For people with sickle cell disease living in rural areas the challenges are greatly amplified. Utilization of services is directly related to socioeconomic conditions that patients face and clinic distance. Patients with sickle cell disease in rural South Carolina without access to comprehensive care, for instance had a higher rate of acute care utilization and readmission. Not surprisingly, patient dissatisfaction often is high due to long wait times for care that is seen as suboptimal. Telemedicine and tele-mentoring are new approaches that harness technology to extend sickle cell disease management expertise from urban centers into rural regions. Dr. Kanter addresses this vexing problem in care access for people with sickle cell disease.

Enhancing Care Within the Institution: Establishing a Sickle Cell Disease Clinic and/or Day Hospital for Comprehensive Care Management

Unpredictable episodes of severe acute pain in childhood that evolve later in life into chronic, often debilitating pain syndromes are the sine qua non of sickle cell disease. Episodes of pain become more frequent in older youth and young adults resulting in frequent ED visits and recurrent hospitalizations. This section of the monograph defines the rationale for DH treatment, outlines preparations, discusses identification of resources, guides planning for implementation, and defines metrics that justify the program and support quality improvement.

Rationale for the Establishment of a Dedicated Unit for Care of Individuals with Sickle Cell Disease

Suboptimal care in EDs for individuals with sickle cell disease is one of the most common complaints by patients and families. The challenge is exacerbated by those ED providers who harbor negative attitudes towards individuals with sickle cell disease often labeling them as “drug seeking” or referring to them by the offensive term, “sickler”. In addition, communication between ED personnel, the primary care physicians and sickle cell specialist is often suboptimal. Adding to the challenge, new regulatory guidelines in response to the opioid epidemic have prompted many EDs and inpatient providers to restrict follow-up opioid prescriptions given at discharge, further compromising treatment of pain episodes.

In addition to providing expert care, DHs create a supportive environment that fosters trust between patients and providers. The first event in the creation of a DH is the emergence of a champion to lead the program; second, identification of resources; third, creation of a plan of action and timeline; and finally, definition of metrics for ongoing assessment and quality improvement.

Taking the First Step: Identify a Champion

The first and most important step in developing a dedicated acute care unit for individuals with sickle cell disease is the emergence of a champion for patients with sickle cell disease at the healthcare facility. This most often is a physician with...
an interest in sickle cell disease but may be any committed HCP. In some cases, the initial champion is a patient or parent who demands improvement in the emergency care of individuals with sickle cell disease by the healthcare facility. In Atlanta, the 12-hour, 7 day-a-week ED-dedicated sickle cell disease unit at Grady Memorial Hospital was championed by a parent who lead the parent/patient group and reached out to the Dr. James Eckman, Director of the Adult Sickle Cell Program, to organize the development of the new unit. Another example is the University of Connecticut Health System where Dr. Biree Andemariam championed the Sickle Cell Program and DH.\textsuperscript{18}

Join Hands with the Community

Ideally, planning for optimal treatment for sickle pain crises and other acute care issues should begin immediately in every health system or community with significant numbers of patients with sickle cell disease. Pressure from patients and their families can prod the health system to action, often after complaints to the administration bring media or local political attention to ED care or a preventable negative outcome for a patient. These opportunities can often be used as leverage to facilitate the establishment of an acute care center.

Develop a Business Plan

Preparation begins with determining feasibility of the program by developing a business plan. The initial step is identifying the nature and extent of services that will be provided in the dedicated acute care area. This must include the types of services that will be available, hours of operation, as well as procedures for smooth interactions with the ED, inpatient areas, laboratory, blood bank and radiology. Existing resources should be identified including staff and space.

The current population served should be estimated including numbers of patients registered in the existing sickle cell program, seen in the ED, admitted to the inpatient services, and seen in other outpatient clinics. Estimates of growth potential include opportunity for increased referrals from the
community, numbers of pediatric patients approaching transition, and estimates of regional population size and travel distances. Often, regional and statewide hospital consortia and departments of health maintain utilization data that can inform population estimates. Partnering with local community-based organizations (CBOs) can also serve as a referral resource for patients not in active comprehensive care.

Financial data to plan feasibility includes an estimate of current costs, charges and reimbursement for emergency department, outpatient, and inpatient services. Baseline data should identify insurance types including Medicaid, Medicare, commercial insurance, and percentages of uninsured in the existing patient population. Potential cost improvement can be estimated from projected decrease in admission rates, reduced length of stay, prevention of unnecessary diagnostic and therapeutic interventions, and increased hydroxyurea use and adherence to therapy.

Get Your Plan Approved
Once data are generated in these preparatory activities, a meeting of all impacted stakeholders should determine if the project will go forward. Advocacy by patients and caregivers, community sickle cell organizations, advocacy groups, funding entities, managed care organizations, and politicians can help make the project a reality. Health system administration, the sickle cell champion, patients/caregivers, impacted healthcare providers, nursing, social services, pharmacy, laboratory services and information technology need to approve both moving forward and the scope of the acute care facility.

Identify Resources, Recruit Stakeholders, and Develop an Action Plan
Approval of the concept of a dedicated acute care facility such as a day hospital initiates detailed planning. A formal planning group of stakeholders must be established that includes health system administration, the sickle cell champion, patients/caregivers/CBOs, impacted healthcare providers, nursing, social services, pharmacy, laboratory services and information technology. This group will determine required space needs, personnel resources, timeline for planning and implementation, finances and cost accounting, as well as interactions between other health system services.

Health system administration usually leads overall plans for allocation of space, financial oversite, administrative structure, and assessment of outcomes. The sickle cell champion, patients and caregivers, impacted healthcare providers, nursing, social services, pharmacy, and laboratory services develop the personnel needs, general methods of operation, patient flow, and outcome quality metrics.

An action plan outlines detailed planning for the acute care facility. Hours of operation and numbers of beds required will determine if the facility will be integrated into an infusion center or observation facility in the emergency department or become a free-standing clinic. Other areas that may support such clinics include ED observation units, bone marrow transplant clinics, and transfusion units. Details of space and allocation will be required to determine patient flow, integration into the sickle cell continuity clinic, transfers to and from the emergency department, procedures for transport to laboratory and radiology facilities, and admission protocols. Procedures, protocols, and storage for medications, opioids, intravenous fluids, blood products and infusion/patient-controlled analgesia pumps need to be defined. The location should be accessible for patients and families with adequate waiting rooms and facilities for families and other care givers.

Personnel requirements include the core physicians and physician extenders, nurses, clerical support, and medical assistants who will staff the acute care facility. Support staff enable providers to focus fully on patient care thereby improving continuity, maximizing efficiency, and reducing provider burnout. Personnel need to be identified to provide social support, psychological interventions, and education. Ideally, a dedicated social worker should be hired, preferably one with a mental health therapy expertise and familiarity with the psychosocial stressors endured by many patients.

Secure and Maintain Funding
A detailed financial plan should be co-developed by the administration and the sickle cell champion. The basis for the financial projections is a cost-saving model based on existing resources. Financial plans often are justified by reducing losses, an important consideration to financial administrators to which clinicians must be attuned. Sickle cell disease
programs often are money-losing cost centers because some segments of the population are uninsured or underinsured leading to inadequate levels of reimbursement. For example, inpatient stays that exceed diagnosis-related group reimbursement produce profound inpatient losses. Unlike inpatient stays, outpatient costs are not bundled so that outpatient losses are lower relative to the inpatient setting. Sickle cell disease programs are often justified by reducing losses rather than increasing profits. These are systemic constraints that must be understood and accepted by the system’s financial leadership. Even modest improvements in average length of stay can translate into large cost savings provided the resources on the outpatient side are sufficiently robust to prevent admission in the first place, reduce the likelihood of a readmission within 30 days, or to insure proper post-discharge clinical follow-up including assessment of social contributors to long lengths of stay (e.g., lack of stable housing). Availability of efficient acute care can facilitate earlier discharge and reduce readmissions, thus providing other indirect cost savings.

Billing and reimbursement from third-party payers should be maximized. Cost centers need to reflect the total financial profile of the sickle cell patient population including revenue from pharmacy, laboratory, and imaging services in addition to direct billing for encounters. Apheresis has the potential to bring in substantial revenue. Consider embedding apheresis into the new center or, if apheresis is “owned” by another service such as transfusion medicine, be certain to include that downstream revenue in the cost center.

Additional external funding sources have been exploited by several sickle cell disease programs to support special sickle cell treatment facilities. Direct state legislative support can be a funding source obtained by advocacy with support of legislators like the Legislative Black Caucus. Private foundations may also support demonstration projects focused on improving healthcare for orphan diseases. Other philanthropic sources to leverage include grateful patient families and private donations. The health system’s business development staff and legislative liaisons should endeavor to identify and secure new avenues of financial support. The unique ability of sickle cell disease care programs to conduct studies of treatment intervention for acute pain episodes and other complications can attract funding from pharmaceutical companies that can indirectly support the patient management infrastructure.34

Identify and Train Personnel

Personnel requirements of sickle cell disease acute care units are challenging. The basic team essential to evaluation and treatment of acute pain episodes are the physician, physician extenders and nurses. Key ancillary support staff includes medical assistants, social workers, mental health specialists, counselors, chaplains, and care managers. Personnel resources for data collection and analysis will facilitate ongoing evaluations of quality improvement. Once staffing needs are outlined, an environmental scan can define the scope of responsibility and job descriptions.

The recruited and trained personnel form the core team in the unit. Recruiting within the health system will facilitate training because such individuals are already familiar with operations. Key to a successful program is developing and maintaining a team with empathy towards individuals with sickle cell disease and their caregivers who also are invested in improving overall care for people living with sickle cell disease. A medical village must also be identified to provide needs for specialty support and ancillary services. Additional champions within this village will ideally come from the other service lines essential for the operation of the sickle cell unit such as the ED, hospitalist groups, hematology/oncology, nursing, transfusion medicine, obstetrics and pharmacy.

Develop Operating Procedures and Individualized Care Plans

Operating procedures must be developed for administration, nursing services, care protocols, and record keeping. Procedures for transferring patients to the ED and inpatient services must also be codified. Protocols for management of medications, opioids, intravenous fluids, as well as environmental issues should be outlined in an operation manual along with assignment of responsibilities. General patient care protocols are needed for all patients cared for in the unit. These care pathways summarize the general medical needs and opioid management issues unique to patients with sickle cell disease.
Ideally, these care pathways should be individualized into care plans for each patient treated in the unit. Plans should outline specific medical problems and ongoing approaches to management. Plans should cover management of acute pain episodes, chronic pain, transfusions, and other ongoing medical issues. Care plans must be multidisciplinary with input from the medical team, social service, pain management specialists, and psychology. Specific individual protocols for opioid use with appropriate monitoring are needed to address management of acute and chronic pain. Ongoing communications with ED staff along with updates or changes to plans are vital to maintaining an effective program.

A mechanism should exist that makes the care plan available throughout the health system and especially in the ED, inpatient units, and primary care clinics. These should be reviewed regularly and modified as the clinical situation changes. Specific protocols are necessary for periods of increased frequency of pain episodes, pregnancies, and medical complications. The care plan should be highlighted in the communications tab of the electronic health record and should include contact information for the primary provider/champion.

A medical community for each patient is developed that includes all the providers required to address primary care, chronic disease management, and specialty needs for individuals with sickle cell disease. Even if existing providers are not well-versed in the management of sickle cell disease, ongoing clear communication and education can quickly enhance their level of comfort. Often, simply reassuring the collaborating providers that they will not be required to coordinate chronic pain and opioid prescription management makes them more receptive to serving as engaged practitioners upon whom the sickle cell program can count.

Follow Metrics to Refine Action Plan and Maintain Institutional Support
Experience with managing dedicated acute care facilities for sickle cell disease demonstrates the importance of collecting metrics in planning, implementing, managing, and improving care. Careful thought is required in developing data management activities to document the impact of the program, justify maintenance and expansion, improve efficiency, and support ongoing quality improvement. Baseline data can be used to justify initiating program services based on projections of utilization and future growth. Data collected during operation can document financial impact, improvement in care, and patient satisfaction.

Baseline data includes number of patients in the sickle cell clinic and health system, as well as the number utilizing the ED and living in the region served. Current insurance profiles of the population and distances from the center are important. Data are needed on current numbers of clinic visits, ED visits, admissions, average length of stay, and 30-day readmission rates. Important baseline clinical data include time to treatment and admission rates in the ED, visit rates per patient, the rate at which health maintenance visits to the clinic are kept, adherence to treatment, and hydroxyurea utilization. Surveys of patient satisfaction, quality of life, social functioning, and economic status should be collected early in implementation.

As the program develops, these parameters should be regularly reevaluated along with ongoing program costs and revenues. Morbidity and mortality rates in the population should also be documented. There should be ongoing assessment of referral sources of new patients including self-referrals, transition from pediatrics, consultations and referrals from the community, primary care, and other specialists.

These data can justify ongoing support for the program by the health system. The data are also used for ongoing quality assessment and improvement in all aspects of operation. Experience in the Grady Health System in Atlanta, Georgia and University of Connecticut Health System in the Greater Hartford region of Connecticut document the critical importance of such activities in justifying, maintaining and expanding those programs. Such data also document and support ongoing quality improvement in services.

Conclusions
Day hospitals utilizing appropriate approaches to managing sickle pain episodes reduce time to pain control, rate of hospitalization, length of stay, and overall costs in adults.15,35-37 Experiences from Atlanta and Hartford demonstrate that integrating
such services into comprehensive care requires a champion who initiates early advocacy and preparation; recruits and organizes adequate resources; develops an action plan and timeline; and identifies appropriate metrics and collection strategies. Care pathways to assure rapid initiation of appropriate pain management are critical to improving patient outcomes and reducing hospitalization rates. Individualized patient-specific comprehensive care plans reduce emergent outpatient episodes, admission rate, length of hospitalization, and readmission rates. Ongoing collection of quantitative and qualitative patient reported data is critical for justifying the program and for cycles of quality improvement that enhance both patient outcomes and program cost effectiveness.

**Utilizing Existing Care Infrastructure: Leveraging Available Capacity by Partnering with Non-sickle cell disease Stakeholders**

Comprehensive care for people living with sickle cell disease has important infrastructure requirements including, but not limited to, a venue for outpatient and inpatient care along with DH/infusion/acute care treatment facilities as alternatives to general ED management. These facilities are manned by key personnel including hematologists and advanced practice providers who coordinate with other providers such as nurses, social workers and pharmacists in the delivery of quality care.

An earlier section of this monograph covers planning for a day hospital and/or acute care treatment facility for patients with sickle cell disease. The expense of creating new facilities for a small patient group with specialized needs can, however, be daunting. In the case of sickle cell disease, some institutions have addressed the problem successfully by leveraging available capacity within the existing medical center infrastructure, such as cancer or hemophilia treatment centers. Table 1 highlights the pros and cons of these approaches to expand care for patients with sickle cell disease.

Another intriguing approach currently being explored is collaboration with Federally Qualified Health Centers for the delivery of quality care to patients with sickle cell disease. Federally Qualified Health Centers generally are in easily accessible community settings. They excel in health maintenance but generally are ill-suited to manage the acute or emergency issues that are integral to the care of people with sickle cell disease. Health maintenance is valuable, however, in preventing some of the co-morbidities that trigger acute issues for patients with sickle cell disease. Moreover, PCPs at FQHCs are well-positioned for surveillance and long-term care of issues such as hypertension, diabetes and other important chronic conditions not connected to sickle cell disease. Federally Qualified Health Centers therefore can be important partners in care delivery for these patients.

**Preparation and Program Construction**

Money, space, and staffing commonly are a triple barrier to the creation of facilities to address the healthcare needs of people with sickle cell disease. Less obvious than lack of space and people are inertia and acceptance of the status quo. More often than malice, belief in the adequacy of existing care derives from a failure to appreciate the challenges faced by patients with sickle cell disease and the degree to which “standard” practice fails to provide quality care. Institutional change is difficult and requires careful planning and coordination.

Leveraging existing infrastructure first requires assessment of where within the institution surplus capacity might exist and the degree to which that capacity is compatible with the care requirements of patients with sickle cell disease. The programs within medical centers most frequently compatible with management of sickle cell disease are comprehensive cancer centers (Figure 4). Oncologists (sometimes) and hematologist/oncologists (often) receive training in management of benign hematologic conditions including sickle cell disease. Most often, such training is not reinforced in subsequent oncology practice, where hematologic disorders overwhelmingly are malignancies. However, a foundation exists that can support high-quality care when sickle cell treatment is added to existing oncology programs.

Bone marrow suppression and associated cytopenias are primary adverse effects of chemotherapy, meaning that red blood cells, neutrophils and platelets are part of the daily dialogue of allied healthcare personnel in oncology centers. Although the focus of laboratory interpretation differs for patients with
sickle cell disease, advanced practice providers and nurses in oncology centers are facile with the elements of blood panels. Moreover, transfusion, a common intervention for sickle cell disease, is an integral part of patient management in oncology centers.

Another model of infrastructure sharing involves hemophilia treatment centers. As a benign hematologic condition, hemophilia is included in the same subspeciality as sickle cell disease. Pain management commonly is a point of overlap between sickle cell disease and hemophilia due to recurrent bleeds into large joints with the clotting disorder.

A key challenge to leveraging infrastructure resources in comprehensive cancer centers or hemophilia treatment centers is convincing leadership to address and remove barriers to sickle cell disease care (Table 2). No financial incentive exists since sickle cell disease treatment programs are not profitable. The argument that quality sickle cell disease care reduces losses associated with management of these patients might resonate with the leadership. In the end, however, the case rests on the humanitarian value of doing the right thing.

In addition, issues related to regulations and charters are important in considering cancer centers or hemophilia centers as sites of sickle cell disease care. The charters of comprehensive cancer centers maintain that the programs exist to treat cancer patients, which is an understandable stipulation to ensure that funds directed toward cancer care and research are used for that purpose. Flexibility exists in these guidelines, but the leadership of the cancer center must be willing to use it. Similarly, an important aspect of funding for hemophilia treatment centers involves use of the 340B

| Table 1. Partnering with Existing Infrastructure to Expand Care for Patients with Sickle Cell Disease |
|-------------------------------------|-------------------------------------------------|-------------------------------------------------|
| **Partner**                          | **Pros**                                        | **Cons**                                        |
| Comprehensive Cancer Center         | ☐ Common at medical centers                     | ☐ High cost value to patient-treatment chairs   |
|                                    | ☐ Familiarity with blood-related issues          | ☐ Scheduled rather than episodic or acute chair use |
|                                    | ☐ Pain management expertise                      |                                                |
|                                    | ☐ Infusion facilities                            |                                                |
|                                    | ☐ Transfusion expertise                          |                                                |
|                                    | ☐ Pharmacy availability                          |                                                |
|                                    | ☐ Laboratory testing                             |                                                |
|                                    | ☐ Patient-treatment chairs                       |                                                |
|                                    | ☐ Psychosocial support services                   |                                                |
| Hemophilia Treatment Center         | ☐ Familiarity with blood-related issue           | ☐ High cost value to patient-treatment chairs   |
|                                    | ☐ Infusion facilities                            | ☐ Uncommon at medical centers                   |
|                                    | ☐ Pharmacy availability                          | ☐ Lack of pain management expertise             |
|                                    | ☐ Laboratory testing                             |                                                |
|                                    | ☐ Patient-treatment chairs                       |                                                |
|                                    | ☐ Management of episodic/acute events            |                                                |
|                                    | ☐ Psychosocial support services                   |                                                |
| Federally-Qualified Health Center  | ☐ Community location                             | ☐ Lack of episodic/acute clinical management expertise |
|                                    | ☐ Low infrastructure and treatment cost          | ☐ Lack of pain management expertise             |
|                                    | ☐ Orientation toward health maintenance           | ☐ Unfamiliarity with blood-related disorders     |
|                                    |                                                | ☐ Lack of patient-treatment chairs              |
|                                    |                                                | ☐ Lack of infusion facilities                   |
Drug Discount Program in which drug manufacturers provide outpatient drugs to eligible health care organizations and covered entities at significantly reduced prices. The intent of the 340B program is to allow covered entities to stretch scarce federal resources as far as possible to reach more eligible patients and provide more comprehensive services. Hemophilia treatment with biologic agents is the centerpiece of hemophilia programs and naturally their operations are aimed at patients with hemophilia. Some flexibility exists but willingness to use it is the key.

As noted in the Introduction, pain management is central to the care of people with sickle cell disease. Pain control is also a common issue for patients with cancer. Opioid administration and management are not intimidating for cancer center personnel even when drug doses substantially exceed those used in routine patient care. Nevertheless, oncology staff are not immune to prejudices around opioid use and misuse that often stigmatize patients with sickle cell disease. Training and education are needed to address the false belief that high opioid requirements are de facto evidence of addiction.

Program Start-up

Convincing medical center leadership of the feasibility of a sickle cell clinical program requires data and a business plan (Table 2). An outline of the nature and scope of the planned services is the first step. New programs most often focus on acute care services with routine clinic care as a subsequent or ancillary component.

Once the scope of the operation is fixed, staffing requirements can be determined. Usually, a physician-champion drives the creation of services. However, the program cannot run without support from other physicians, advanced practice providers, nurses, and administration. A plan for identifying and hiring new staff or reassigning existing staff is vital. Furthermore, training to create both competence and confidence for providers new to sickle cell disease care is essential to success. Plans to integrate ancillary services including laboratory, blood bank,
and radiology are important to program operation. Fortunately, most such services are integral to cancer centers and hemophilia programs abrogating the need for extensive restructuring.

A crucial difference between cancer care and acute management of sickle cell disease is the ratio of planned versus urgent infusion visits. Most treatment chairs in cancer infusion suites are filled with patients who are scheduled days or weeks in advance. In contrast, acute sickle cell pain episodes are sporadic and unpredictable, creating a problem when planning availability of treatment chairs. Chairs may be empty on some days while on others the number of patients exceeds capacity. The latter issue requires coordination for rerouting patients to the ED. Empty chairs highlight a key financial issue that overhangs the arrangement. Chemotherapy infusions and other cancer treatments fill chairs with patients whose treatment is profitable for the center. Each chair occupied by a patient with sickle cell disease is however a potential net loss for the institution. Frank discussion is needed around the issue of profits and losses associated with reassignment of resources.

The nature and extent of services that will be provided in the dedicated acute care area must also be defined. Considerations include the types of services that will be available, hours of operation, as well as procedures for smooth interactions with the ED, inpatient areas, laboratory, blood bank and radiology. Existing resources should be identified including staff and space.

The active sickle cell patient population should be estimated including numbers of patients seen in the ED, admitted to the inpatient services, and seen in outpatient clinics. Estimates of growth potential should include the likelihood of increased referrals from the community, the number of pediatric patients approaching transition, estimates of regional population size and travel distances. Often, regional and statewide hospital consortia and departments of health maintain utilization data that can inform population estimates.Partnering with local CBOs can also serve to estimate the number of patients not currently in comprehensive care.

Financial data to plan feasibility includes estimating costs, charges and reimbursement for current emergency department, outpatient, and inpatient services. Baseline data should identify insurance types including Medicaid, Medicare, commercial insurance, and uninsured percentages in the current patient population. Potential cost improvement can be estimated from projected decreased rate of ED visits and hospitalizations, reduced length of stay, prevention of unnecessary diagnostic and therapeutic interventions, and increased hydroxyurea use and adherence to therapy.

Once data are generated in these preparatory activities, the impacted stakeholders should meet to determine how the project will proceed (Table 3). Advocacy by patients and caregivers, community sickle cell organizations, funding entities, managed care organizations, and politicians can help make the project a reality. Buy-in to the acute care facility should come from health system administrators and the sickle cell disease champion, along with representatives of nursing, pharmacy, radiology, laboratory, ED, and inpatient services.

Table 3. Leveraging Available Health Center Resources for Sickle Cell Disease Care: Key Partners

<table>
<thead>
<tr>
<th>Direct Care</th>
<th>□ Medical Staff</th>
<th>□ Nursing Staff</th>
<th>□ Emergency Department</th>
</tr>
</thead>
<tbody>
<tr>
<td>Care Support</td>
<td>□ Social Services</td>
<td>□ Pharmacy</td>
<td>□ Laboratory Services</td>
</tr>
<tr>
<td></td>
<td>□ Blood Bank</td>
<td>□ Pain Management</td>
<td></td>
</tr>
<tr>
<td>Administration</td>
<td>□ Department Chairs</td>
<td>□ Health System</td>
<td>□ Hospital</td>
</tr>
<tr>
<td></td>
<td>- Medicine</td>
<td>□ - Oncology/ Hematology</td>
<td></td>
</tr>
<tr>
<td></td>
<td>- Nursing</td>
<td>□ - Nursing</td>
<td></td>
</tr>
<tr>
<td>External</td>
<td>□ Political leaders</td>
<td>□ Community-based</td>
<td></td>
</tr>
<tr>
<td></td>
<td>□ Patients/families</td>
<td>organizations</td>
<td></td>
</tr>
<tr>
<td></td>
<td>□ Funding entities</td>
<td>□</td>
<td></td>
</tr>
</tbody>
</table>
Program Execution

Although adequate planning expedites program execution, the process is far from simple. The personnel needed to care for the new patients with sickle cell disease come from reassignment of existing personnel or new hires. Reassignment taps people familiar with operations within the institution and can be challenging due to potential shortages created for existing oncology or hemophilia patients. Planning and careful coordination help avoid impasses that can sabotage a new effort before it begins. New hires circumvent this issue. Both however will need training in management of people with sickle cell disease. The depth and breadth of the training will vary but cultural sensitivity should be an integral component. An advanced practice provider is the hub of most sickle cell disease treatment programs and should be in the first wave of recruitment.

Care pathways are essential to the proper management of patients with sickle cell disease. A generic pain management protocol should be established as a template to be personalized for each patient. A personalized treatment plan can avoid frustration for both the patient and care personnel. Operating procedures should be codified to cover the gamut of issues around acute management of people with sickle cell disease including fluid administration, transfusion, management of fever, and radiographic investigation.

The quality and severity of vaso-occlusive pain varies greatly for patients with sickle cell disease along with the analgesics to which they respond. Unfortunately, opioids are at the epicenter of a public health emergency in the United States. These drugs are essential for pain management by many patients with sickle cell disease and most handle the medications without misuse or abuse. However, patients with sickle cell disease are caught in the whirlwind around opioid management and restriction. Clear documentation of patient history and needs, along with an individualized management that is adjusted based on response and can minimize pain (both physical and psychological), is essential. Pain specialists should participate in management whenever possible.

Data capture for patients treated in the new center is vital. A database beyond the standard electronic medical record can capture information needed to demonstrate program success. Programs without such benchmarks will be severely handicapped in measuring outcomes. A patient database could also provide anonymized information to national sickle cell disease registries that are under construction. Data sharing contributes to the long-term improvement in patient care both locally and nationally.

Metrics/Measurement

Table 4 lists some of the parameters that can be assessed to determine the success of the sickle cell treatment program. Others are possible but should fit the SMART criteria: Specific, Measurable, Achievable, Relevant and Time-bound. Essential assessments without SMART characteristics are patient and staff satisfaction. Continual review and adjustment will improve the quality of the program.

Conclusions

Integrating a care program specific to sickle cell disease into an existing system can markedly improve care for people living with the condition. An innovative/creative approach is sometimes needed to

<table>
<thead>
<tr>
<th>Table 4. Leveraging Available Health Center Resources for Sickle Cell Disease Care: Success Metrics and Measures</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Metrics/Measurement</strong></td>
</tr>
<tr>
<td><strong>Quality of treatment</strong></td>
</tr>
<tr>
<td>□ Decreased time to first dose</td>
</tr>
<tr>
<td>□ Improvement in pain</td>
</tr>
<tr>
<td>□ Decreased admissions</td>
</tr>
<tr>
<td>□ Decreased readmissions and return ED visits</td>
</tr>
<tr>
<td><strong>Access to acute treatment</strong></td>
</tr>
<tr>
<td>□ Increased visits to day hospital/infusion center</td>
</tr>
<tr>
<td>□ Increased observation visits</td>
</tr>
<tr>
<td><strong>Continuity of care</strong></td>
</tr>
<tr>
<td>□ Immunizations</td>
</tr>
<tr>
<td>□ Disease-modifying therapy</td>
</tr>
<tr>
<td>□ Decreased hospitalization rate</td>
</tr>
<tr>
<td><strong>Patient Satisfaction</strong></td>
</tr>
<tr>
<td>□ Complaints</td>
</tr>
<tr>
<td>□ Surveys</td>
</tr>
<tr>
<td><strong>Costs</strong></td>
</tr>
<tr>
<td>□ Reduced cost to institution for sickle cell patient care</td>
</tr>
</tbody>
</table>
integrate into existing infrastructure (e.g., integration into an oncology program with specifically allocated chairs for patients with sickle cell disease). With any new service, champions/advocates are essential to convert a good plan into a real solution. Patients are persuasive advocates, giving voice to difficult issues that they face. When embarking on such an endeavor, a perfect plan should not impede implementation of a good one—take what is available and implement it. Most importantly, approach adversity with patience, using each challenge as opportunity to educate and improve the sickle cell program.

**Transitioning Adolescent Care: Structuring Transition Between Pediatric and Adult Care Settings**

A transition program that facilitates the move from pediatric to adult care for adolescents and young adults with sickle cell disease is critical to optimal long-term health outcomes. Indeed, mortality of patients with sickle cell disease older than 18 years increases by 2- to 3-fold relative to younger patients. The rate of acute care encounters (ED visits and inpatient stays) also increases markedly in patients aged 18–30 years relative to younger cohorts. Because most children born with sickle cell disease in high-resource countries survive to adulthood, the primary impact of morbidity and mortality has shifted to adults, making the transition to adult-focused care a high-risk period for death. As patients transition from childhood to adulthood, chronic comorbidities increase including chronic pulmonary disease, nephropathy, and cardiovascular complications. A coordinated effort around care transition can mitigate the dire health consequences suffered as patients cross the threshold between the pediatric and adult worlds. Recognition of the challenges faced by adolescents and young adults is the first step toward solutions.

**Overarching Issues Specific to Pediatric/Adult Transition**

When considering transition from pediatric- to adult-based sickle cell disease care, the meaning of the word “transition” must be clear. Transition is an orderly, coordinated shift in patient care from a pediatric program to an adult program. A shift in patient care that is not orderly and coordinated is a transfer, not a transition (Figure 5). Transfers generally result in poor patient care compared to transition with a key factor being the difficulty patients face in crossing the care chasm without guidance or assistance. Because sickle cell disease is a life-long chronic illness whose characteristics change between childhood and adulthood, care continuity is essential. Management and prophylaxis...
programs developed during early care should be carried forward into the adult setting as appropriate. Anticipation of issues that emerge in adulthood is an important component of transition.

Limited guidance exists on transitioning patient care from pediatric to adult settings for people with sickle cell disease. Adult-focused programs are commonly driven by a single provider which contrasts with pediatric programs that often have more resources, a broad team (e.g., nurses, social workers) and usually are more “nurturing” for patients. Furthermore, pediatric providers have a well-rounded perception of patients with sickle cell disease due to extensive clinical contact around issues such as penicillin prophylaxis and vaccinations which create a rounded and holistic view of patients. In contrast, institutional experience with sickle cell disease for adult providers often is based on a small sub-population of patients with high ED utilization and frequent inpatient stays, fueling a perception of high opioid use, noncompliance, and high readmission rates. Data are key to creating an accurate picture and changing institutional perceptions of young adult patients with sickle cell disease. For instance, at the Children’s Hospital of Philadelphia, of 179 patients with sickle cell disease between the ages of 18 and 22 years, only 8 patients (4.5%) had more than 10 hospital admissions per year and only 2 patients (1.1%) spent greater than 50% of days on documented outpatient opioids.

Poor communication between pediatric- and adult-focused programs, CBOs, and patients/families is a frequent issue that complicates transition planning and execution. Poor understanding of coping and support needs of young adults as they move through transition is another obstacle. In addition, patients often have only a vague understanding of transition as well as limited insight into their basic medical history. A “bridge” is needed to allow patients to experience and understand the new adult medical care model. Communication and education are needed for pediatric patients to make the transition to adult-focused care.

Essential structural components to transition include a pediatric care program, an adult care program, and a bridge between the two (Figure 5). Commencement and completion of the transition process is variable meaning that institution-specific guidelines must be developed. Patients, families, and pediatric providers develop a special bond over years, derived from care that often stretches from infancy. Relinquishing that care to strangers of unproven mettle can be trying for all. No substitute exists for un rushed time during which patients, families and adult providers develop familiarity and trust.

**Barriers Unique to or High Profile in Transition**

Transition occurs during, and is complicated by, one of life’s greatest challenges: adolescence. Even for unaffected children, the physical and emotional changes wrought by adolescence can smash childhood tranquility into painfully jagged fragments. For adolescents struggling with sickle cell disease that often is morphing in character and intensity, the teenage years can be a nightmare. Adding transition to this chaotic climate can approach cruel and unusual punishment for patient and family. Patience and resolve by healthcare providers are vital to navigating these rocky rapids.

A key consideration in pediatric to adult transition is the age at which the issue should be addressed with patients and families. Initial discussion should be part of visits during pre-adolescent years. Social workers from pediatric and adult teams determine “readiness” (which may not always be patient-dependent: the adult care providers also need to be ready for the new patient). Transition should be carefully thought out and paced.

The optimal age is not well defined, and no clear age-dependent triggers exist for transition. Often, the transition age is payer driven but remains highly variable, with no standardization. A common and unfortunate result is complexity and confusion for patients and families. Medicaid programs in some states such as Texas require a transition plan, but this is not a uniform mandate across states.

Patient separation from their pediatric provider and lack of preparedness of the adult healthcare system are major obstacles to successful transition. Brain injury and neurocognitive impairment are important comorbidities in sickle cell disease producing developmental challenges that can also complicate transition readiness. Suboptimal
adult care programs, including a lack of adult care providers, a deficit in provider readiness (only about one third of general internists feel comfortable being the primary provider for patients with sickle cell disease\textsuperscript{49}), and a dearth of nurse coordinators, psychologists, patient navigators/coordinators, make transition unattractive to patients and parents. Adult subspecialty providers such as cardiologists and nephrologists must also be considered as part of transition planning.

Consequent anxiety for patients and families as well as patient depression are significant concerns. Social support therefore is an important component of transition success. Other barriers include the lack of systematic approaches or processes to track patients through transition, lack of a standard definition of successful transition, lack of third-party payer care/case management, and lack of knowledge around support for patients who fail transition readiness but are adults. In addition, many patients need coaching on personal accountability and skills for navigating the healthcare system.

Transition Resource Planning: Pediatric and Adult Programs

Figure 6 highlights the conceptual framework of transition and the key personnel and other factors that must be integrated into planning. Facilitators are vital to success and include program champions, hospital administration (chief medical officers are particularly important to program administrative ownership), medical staff (including psychiatry/psychology), nursing staff as well as patients and their caregivers. Table 5 lists these and other important stakeholders including community advocacy groups (foundations, churches/faith-based organizations), third-party payers as well as public or political champions. Input from stakeholders is key to creating a shared vision of success.

Resource planning is vital to success when building a sickle cell disease transition program. This includes a business plan, a program and services plan, plans for addressing personnel requirements as well as a needs assessment for space. Staff requirements include dedicated medical providers, advanced practice providers, social workers, patient navigators, care coordinators/managers, and a support mechanism for patients (e.g., community health worker, peer mentor, etc.). Other potential personnel ideally are multi-level providers (i.e., medicine/pediatrics), educators (disease self-management, educational modules), family medicine, mental health, vocational/occupational specialists, sexual reproductive health specialist, and genetics counselor. Although this comprehensive list is daunting, programs usually begin with a minimal provider contingent and build out as success grows.

Transition Resource Planning: Specific to Adult Program Partners

On the adult side of the transition bridge, solicitation and recruitment of adult providers to care for transitioning patients is paramount. Providers who actively care for sickle cell disease patients are a good starting place. Hematology/oncology programs emphasize hematologic malignancies. However, such programs often have a provider who focuses on benign hematology who may be open to adding sickle cell disease to the treatment portfolio. A pilot initiative involving a small number of patients can be useful to resolve bumps before a large patient influx.

Another pivotal step is reaching agreement with adult providers on key requirements for transition. A phased startup approach using age- or disease-based prioritization is a reasonable first step. For example, priority might be given to transitioning adult patients in the pediatric program or patients with milder disease, keeping complex patients on the pediatric side while the adult providers become more comfortable with management of sickle cell disease. Tiered efforts build confidence of patients and providers, as well as program supporters and champions.

The transfer of key patient information at handoff is also essential to transition. In this age of early hydroxyurea treatment, some patients with sickle cell disease have pediatric courses that are barely distinguishable from unaffected children. Unfortunately, others have major challenges including strokes, acute chest syndrome and frequent vaso-occlusive pain crises. Chronic transfusion treatment with its attendant comorbidities is a common consequence of these clinical challenges. The adult providers in the transition program must receive key information from the pediatric medical record, such as data related to chronic exchange transfusion, as well as management plans to avoid potentially dangerous treatment lapses.
Transition Initiation

Creation of an institutional policy around the age for transition initiation is the first step in building a program. As noted earlier, an optimal age for transition from pediatric to adult sickle cell disease care is not well-defined. Discussions of transition with patients and families should start early on before the process truly begins to avoid the perception of an abrupt cutoff of care. Patients and families commonly view late adolescence as a time to consider transition. Many clinicians feel however that initial discussion of transition at around age 14 years of age is appropriate. An early start on the transition pathway allows patients to make decisions and advocate for themselves, as well as take responsibility for medication adherence and other aspects of care. Moreover, parents can be weaned from “overprotective” behaviors derived from difficulty letting go. Pediatric providers may also request that parents wait in lobby until the end of exams/visits to allow individual, confidential discussions and HEADSS assessment (Home, Education, Activities/employment, Drugs, Suicidality, and Sex).

Payer coverage issues often are also linked to patient age (e.g., 18 years) placing additional constraints on care transition. In any case, patients and families who are forewarned about transition can better cope with the potential stress inherent to the process.

An educational program around transition is one way to forewarn and forearm patients and families. Transition to the adult care world is scary and mysterious for all patients and those with sickle cell disease are no exception. Transition can be...
laden with anxiety and angst. An education program that commences before transition can smooth the process. An emphasis on positive features of transition, such as the child becoming a big person like their siblings, relatives and friends sends a positive and hopeful message. Even today, patients with sickle cell disease at times are told they will not live long. Preparation for transition is a clear message to the contrary.

Ideally, the initial discussion of transition should be in the pediatric facility with the prospective adult providers attending as guests. New faces seen in a familiar environment eases stress for patients and families while allowing the adult providers to see how patients and families interact with their long-standing healthcare team. This introduction should occur months or years before the patient’s first visit to the adult facility. Vital information for patients and families includes the location of the adult facility and detailed instructions on how to reach the sickle cell disease clinic. Clinic phone numbers should be provided along with details on information services at the adult facility. Check-in calls from the adult clinic should occur prior to the first visit and follow up calls should be made if the visit is missed. Patients can be lost for months or years due to failures at the first hand-over visit, sometimes with disastrous consequences.

### Transition Execution

A transition advisory committee composed of healthcare providers, patients/families, and advocates can give useful insight into challenges and successes of transition. In addition, a real-time tracking program is helpful for monitoring individual patients, opening the possibility of midcourse correction to the transition process.

---

### Table 5. Resource Planning and Recruitment for Pediatric to Adult Transition

<table>
<thead>
<tr>
<th>Stakeholders</th>
</tr>
</thead>
<tbody>
<tr>
<td>☐ Patient and family first and foremost</td>
</tr>
<tr>
<td>☐ Hospital team of peers, others doing transition</td>
</tr>
<tr>
<td>☐ Divisional leadership</td>
</tr>
<tr>
<td>☐ Hospital administration</td>
</tr>
<tr>
<td>☐ College-bound patients—college campuses, office of disability on college campuses</td>
</tr>
<tr>
<td>☐ 3rd-party payers</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Champions</th>
</tr>
</thead>
<tbody>
<tr>
<td>☐ Someone you need in a specific role to help you accomplish a specific task</td>
</tr>
<tr>
<td>☐ CBOs</td>
</tr>
<tr>
<td>☐ Political leaders</td>
</tr>
<tr>
<td>☐ Public figures</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Potential Funding Sources</th>
</tr>
</thead>
<tbody>
<tr>
<td>☐ Patient insurance, comprehensive care</td>
</tr>
<tr>
<td>☐ 3rd-party or hospital funding</td>
</tr>
<tr>
<td>☐ Private donations/foundations/philanthropy</td>
</tr>
<tr>
<td>☐ Grants—federal or private</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Staff Requirements</th>
</tr>
</thead>
<tbody>
<tr>
<td>☐ Nurse/nurse practitioner</td>
</tr>
<tr>
<td>☐ Social Worker</td>
</tr>
<tr>
<td>☐ Care coordinator</td>
</tr>
<tr>
<td>☐ Support mechanism for patients—community health worker, peer mentor, etc.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Other</th>
</tr>
</thead>
<tbody>
<tr>
<td>☐ Space, approval for the space</td>
</tr>
<tr>
<td>☐ Education materials for transition readiness</td>
</tr>
<tr>
<td>☐ Transition process tracking resources</td>
</tr>
<tr>
<td>☐ Other infrastructure</td>
</tr>
</tbody>
</table>
Patient data collection, both at baseline and over the course of transition, is vital to assess program success. Careful planning is required to create a data management program that documents the impact of the transition effort, justifies maintenance and expansion, improves efficiency, and supports ongoing quality improvement. Baseline data can be used to justify initiating the program while data collection during operation of the transition program documents financial impact, improvement in care, and patient satisfaction.

A database beyond the standard electronic medical record should be created to capture information needed to demonstrate program success. Aggregate data along with trends and statistical considerations are powerful tools for analysis of program activities and execution.

**Success Metrics**

Measuring important components and outcomes related to transition is important for stakeholder assessment of program success and to highlight needed adjustments. Table 6 lists some important measures including first visit to the adult clinic, return visits to the pediatric clinic post-transition, ED visits at either adult or pediatric facilities and hospitalizations at either facility. Other important measures include the number of patients transitioned or lost during transition, continuation of scheduled transfusions, continuation of maintenance medications and use of opioid analgesics. Satisfaction reports from patients and staff are also important metrics.

### Table 6. Pediatric to Adult Transition: Assessment of Program Success

<table>
<thead>
<tr>
<th>Individual Patient Measures</th>
<th>Other Measures</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pediatric Encounters</td>
<td>Adult Encounters</td>
</tr>
<tr>
<td>ED visits</td>
<td>First clinic visit</td>
</tr>
<tr>
<td>Hospitalizations</td>
<td>ED visits</td>
</tr>
<tr>
<td>Clinic visits</td>
<td>Hospitalizations</td>
</tr>
</tbody>
</table>

Conclusions

Successful transition from pediatric- to adult-based care is critical to maintain continuity and quality of care for patients with sickle cell disease. Important challenges include when and how to institute transition, lack of adult providers, poor communication between pediatric and adult sickle cell disease care programs, and emotional attachment of patients/ families to their providers in the pediatric setting.

Essential structural components to transition include a pediatric care program, an adult care program, and a bridge between the two. The transition process is variable and therefore institution-specific guidelines must be developed. Important components of the transition program include stakeholders (e.g., patients/family, HCPs, administration, and payers), funding sources, staff (e.g., nurse/nurse practitioner, social worker, and care coordinator), physical space for the program, educational materials for transition readiness, and transition process tracking resources. The program’s success should be assessed by key metrics including patient/family feedback, ED visits, hospitalizations/re-hospitalizations, treatment adherence, and cost. A robust transition program can positively influence a patient’s clinical course for years and even decades.

**Expanding Care Outside the Institution: Building a Community Outreach Model to Extend the Reach of Care**

Healthcare providers with specialized knowledge, experience, and insight are critical to the manage-
ment of complex, chronic disorders. Experience and insight are developed only with frequent patient interactions. The challenge is magnified for rare disorders, which can range from ultra-orphan conditions like paroxysmal nocturnal hemoglobinuria to “more common” orphan disorders like sickle cell disease. Not surprisingly, specialists advanced in the care of rare disorders are usually found at medical centers in large urban areas. To enhance care outside of the institution, it is necessary to build a path to share both the specialized knowledge about the disease as well as the experience with patient management.

Despite new therapies and improved childhood survival, sickle cell disease still limits life expectancy through a myriad of chronic complications. Care provided by specialists familiar with sickle cell disease management is critical if patients are to benefit optimally from current and evolving therapies. Many people living with the disease reside in urban areas such as New York and Chicago where specialists are often available. However, for those living in rural areas, most often in Southern states, the search for knowledgeable providers who deliver quality, evidence-based care often is daunting. Routine travel of several hours to reach sickle cell treatment specialists often strains the social and economic wherewithal of people who may be socio-economically challenged, adding to the health disparities of their disease.

Fortunately, evolving technology is being harnessed to address and mitigate the healthcare chasm between people in urban and rural regions of the country. Figure 7 shows three ways to decentralize sickle cell disease expertise to benefit people in outlying, underserved areas. One is tele-mentoring in which the expert holds online video conferences with providers (typically physicians, advanced practice providers, nurses and social workers) who then deliver patient care at remote locations. In this model, the expert is an adviser and does not provide direct medical care. Instead, the model, based on the ECHO (Extension for Community Healthcare Outcomes) program, uses a case-based teaching approach with additional didactics provided. Multiple remote providers can interact with the expert through group discussion around individual patients.

Figure 7. Approaches to Extend Sickle Cell Disease Care Beyond the Institution
tele-mentoring mimics subspecialty rounds at medical centers, providing additional educational opportunities. With this approach however, the quality of patient care still depends on the skill and knowledge of the local provider. The expertise of sickle cell disease specialists often is intangible and subtle insights can be lost in translation.

In contrast, telemedicine involves interaction of the expert with both the local provider and the patient. As Figure 7 illustrates, this more dynamic interaction allows the expert to evaluate the patient directly and possibly uncover disease-related issues that the local healthcare provider may have missed. Real-time interactions amplify effectiveness in complex conditions like sickle cell disease where multiple organ systems are affected and interact. Telemedicine usually requires a presenter (often a nurse or medical assistant) where the patient is located to facilitate the use of the equipment.

Another way in which telemedicine can be used is within a hub-and-spoke approach, where the expert is credentialed at the outlying facilities, provides medical care, and supervises mid-level practitioners. The hub-and-spoke model can have greater reach than telemedicine alone by expanding care to outlying facilities where an advanced practice provider can give hands-on care on a daily basis, whereas telemedicine clinics are usually more infrequent. An important caveat is that the hub-and-spoke model places greater responsibility on the expert who becomes a real provider through a virtual connection.

Building a program to extend healthcare is daunting. Clarity around strategy and diligence concerning tactics are vital. Identification of local or regional stakeholders, collaboration with ancillary care teams and other approaches that broaden access are crucial to building an effective outreach system. Other factors that can influence a program’s viability include political or community champions, newborn screening programs and associated follow-up activity, as well as private and government-sponsored third-party payment programs.

The South Carolina Sickle Cell Network (SC)², a hub-and-spoke model, provides insight into successful program development. Newborn screening data showed that a substantial number of people with sickle cell disease in South Carolina live in rural regions. However, before creation of (SC)², the three main academic centers in the state had sickle cell disease pediatric programs, but none of them had programs for affected adults. Adults with the disease thus were underserved throughout the state, often seeing primary care doctors, community oncologists or only obtaining care from the ED regardless of whether they lived in the city or in the country. Effective redress required a program that fully assessed the situation and was designed to reach all areas.

**Preparation**

There are several issues to address and barriers to anticipate when expanding sickle cell disease care outside of the main institution (Table 7). Data are key to any new endeavor including the creation of a program to extend quality healthcare into rural America. Data can define the need and identify resources to address that need. As clear, unequivocal facts, data can be the rallying point for supporters, champions and allies.

Understanding sickle cell disease demographics within a state is a key first step in program planning. In the US, Sickle Cell Disease Newborn Screening (NBS) exists in all states and is key to under-

<table>
<thead>
<tr>
<th>Table 7. Extending Sickle Cell Disease Care Beyond the Institution: Overcoming Barriers</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Issues to Address</strong></td>
</tr>
<tr>
<td>□ Patients live far from academic</td>
</tr>
<tr>
<td>center (therefore, no care, ED</td>
</tr>
<tr>
<td>didn’t know what to do, etc.)</td>
</tr>
<tr>
<td>□ No insurance or local SCD</td>
</tr>
<tr>
<td>specialists</td>
</tr>
<tr>
<td>□ Finding affected individuals</td>
</tr>
<tr>
<td>□ Finding interested stakeholders</td>
</tr>
<tr>
<td></td>
</tr>
<tr>
<td></td>
</tr>
</tbody>
</table>
standing patient proximity to urban centers. NBS programs are administered by state-designated entities meaning that information can be acquired only through individual state programs. Currently, there is no national NBS database and many states expunge the data every few years. There are no national registries for sickle cell disease at present and data surveillance has only been done in California and Georgia through CDC-sponsored grant projects. Thus, it can be a challenge to characterize and quantify the local populations.

In South Carolina, a needs assessment was performed to address questions around adult access to care through payer information gathered from the South Carolina Revenue and Fiscal Affairs Office, which keeps health statistics. Statewide administrative data provided key information on acute care utilization during a defined 12-month period (2012) and was shared with state and regional service providers, managed care companies, and policy makers to identify and highlight gaps in care and inform policy around improvements. Information on ED visits and hospitalizations through patient-based uniform billing data were analyzed for acute care utilization and 30-day readmission rates were stratified by patient age, region, and expected payer. These data identified “hot spots” of need that could be targeted for intervention (Figure 8). Following the identification of local hospitals that frequently evaluated patients with sickle cell disease in the emergency departments and inpatient units, specific hospital-based data could be obtained.

Rallying stakeholders to the cause of improved care for people with sickle cell disease should be an early action in the care expansion effort. Table 8 highlights stakeholders key to the mission of providing quality care for people with the disease. Community hospitals are important to people who live far from urban hubs and academic centers. These hospitals often are criticized for poor care while simultaneously shouldering the high cost of care associated with inexpert management, creating an environment unpleasant both to patients and providers. High costs are also an issue for third-party payers. Bringing sickle cell disease management expertise into rural communities is in the interest of community hospitals and payers as well as patients.

Political champions and CBOs within a state can powerfully influence healthcare both by opening access to key decision-makers and by raising the level of public awareness. Social media is a major new force that shapes awareness and public opinion and is a vehicle with which many younger
people, including those with sickle cell disease are facile. Alliances and publicity can help blunt the impact of people who harbor negative or even frankly hostile attitudes toward those living with sickle cell disease.8,59,60

The South Carolina needs assessment confirmed that many individuals with sickle cell disease lived in rural areas and often relied on community hospitals without expertise in sickle cell disease. These data demonstrated very high rates of acute care use and readmission (Figure 8). A discrepancy often exists between pediatric and adult health outcomes in sickle cell disease and is due in part to the paucity of providers willing and able to treat adults living with the disease.61,62 The issue is magnified for people in rural areas far from academic centers where transportation options are limited.53 Routine travel of several hours to reach urban specialists is not sustainable.

Initial efforts through pilot projects often can garner more early support than a full-blown networking effort. Starter programs allow the probing of barriers including local credentialing for the expert and hiring of local staff. Choosing the best networking program is also important. South Carolina chose a hub-and-spokes care delivery model, with Medical University of South Carolina as the hub of sickle cell disease expertise. The spokes provided a local medical home and advanced practice provider-supported clinics, using Telehealth support as needed. The goal was to have sickle cell disease care clinics at each spoke with small “day hospital” care facilities that supported laboratory services, intravenous fluids, pain medications, transfusions, and hospital follow-up.

Facilitators, including supporters within the hub hospital, are crucial to establishing a treatment network. In South Carolina, important support came from the state-run telehealth program, Duke Endowment (funding), and CBOs. Individuals with sickle cell disease and their families also served on a community advisory board to give life and voice to patient needs that might otherwise have been abstract facts and dry statistics. Nothing substitutes for the human face of illness.

Government programs are another potential pillar to support expanded care for people with sickle cell disease. Medicaid is the insurance network for many people with the disease. Some Medicaid programs have health maintenance organization that can be recruited to improve care. In addition, Departments of Health through programs for Children with Special Healthcare Needs can be important allies.

Recruitment/Resources
The next phase of network creation is site recruitment and determination of resource needs. Important site considerations include location and whether the site can be payer-directed or region-directed. Space is a premium in most medical facilities, including those in rural regions. Carving out areas dedicated to treatment of sickle cell disease requires patience and negotiating skill. Identifying allies in the local institutions is vital, including ED medical directors, nursing staff and pharmacists. At the hub hospital, administrators and program directors can facilitate program development. More broadly, medical societies (e.g., the American Society of Hematology and the American Society of Pediatric Hematology/Oncology) and Medicaid medical directors can be useful allies. Support can possibly be garnered through state
departments of public health, hospital in-kind funds, foundations or third-party payers. Demonstration projects by the Health Resources and Services Administration of the US Department of Health and Human Services has supported networks, as well.

Staff requirements for the program are extensive, and includes nurses, pharmacists, primary care providers, and information technology support (Table 9). Establishment of a stakeholder advisory board is another important component of care extension. Advisory board stakeholders include individuals with sickle cell disease and their families, key care providers, third-party payers, state government and Department of Health and Environmental Control members, and CBO members.

Action Planning
The next steps in expanding care outside the institution involve program implementation and measurement of success. Data specific to individual hospitals are needed. Each spoke is unique in its patient and staff composition, operational structure and relationship to the hub. Data from multiple spokes integrated with that from the hub can form a nidus for a statewide patient registry. Standardized data templates and storage procedures which are key components needed for information sharing across a network are also requirements for a registry. Programs start slowly due to builds around credentialing, identification of space, hiring staff and assigning responsibilities. Lessons learned with early adopter spokes can be applied to those that come onboard later.

Metrics/Measurement
Program success and continuation ultimately rest on data. Pre-specified metrics and milestones therefore are vital to long-term program viability. As outlined in Table 10, important metrics include the number of spokes/clinics established, patient access (who and how many patients are seen and how often) as well as the capacity of the various spokes/clinics (what can be done at those locations). Data on use of ED versus day hospital for acute care and 30-day readmission rate gauges the impact of care programs on patients as well as

Table 9. Extending Sickle Cell Disease Care Beyond the Institution: Staff Requirements

<table>
<thead>
<tr>
<th>Measure</th>
<th>Metric</th>
</tr>
</thead>
<tbody>
<tr>
<td>Nurse Practitioner</td>
<td>Nurse/public health nurse</td>
</tr>
<tr>
<td>Social worker</td>
<td>Human services coordinator</td>
</tr>
<tr>
<td>Case manager</td>
<td>Patient coordinator for spokes</td>
</tr>
<tr>
<td>PCP (ideal)</td>
<td>Family medicine resident rotations</td>
</tr>
<tr>
<td>Ideal mental health services</td>
<td>Physical therapy</td>
</tr>
<tr>
<td>Palliative care</td>
<td>Pharmacy</td>
</tr>
<tr>
<td>Pain medicine expert/anesthesiologist</td>
<td>IT support (comes with telehealth?)</td>
</tr>
<tr>
<td>EMR/data</td>
<td>Quality improvement person</td>
</tr>
<tr>
<td>Administrator – policy/procedure/data</td>
<td>Lab/imaging</td>
</tr>
<tr>
<td>Data coordinator</td>
<td></td>
</tr>
</tbody>
</table>

Table 10. Extending Sickle Cell Disease Care Beyond the Institution: Success Metrics and Measures

<table>
<thead>
<tr>
<th>Measure</th>
<th>Metric</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of functioning care clinics (SPOKES)</td>
<td>ED/acute care use</td>
</tr>
<tr>
<td></td>
<td>Telemedicine</td>
</tr>
<tr>
<td></td>
<td>Cost</td>
</tr>
<tr>
<td>Disease-modifying therapy</td>
<td>Hydroxyurea</td>
</tr>
<tr>
<td></td>
<td>Transfusion</td>
</tr>
<tr>
<td>Education</td>
<td>Echo attendance</td>
</tr>
<tr>
<td></td>
<td>Symposium attendance</td>
</tr>
<tr>
<td>Patient engagement and care coordination</td>
<td>New referrals of previously unaffiliated patients</td>
</tr>
<tr>
<td></td>
<td>Individualized pain plans</td>
</tr>
<tr>
<td>Community engagement</td>
<td>Creating new educational symposiums</td>
</tr>
<tr>
<td></td>
<td>Working with CBO to establish case management</td>
</tr>
<tr>
<td>Registry</td>
<td>Start-up</td>
</tr>
<tr>
<td></td>
<td>Enrolled patients</td>
</tr>
</tbody>
</table>
costs to institutions. Establishment of rational opioid management policies across various spokes (same prescribing policies at each location) is another important metric of care quality. Implementation of preventative programs such as prophylactic penicillin and disease-modifying therapies also reflect program success. Finally, overall patient assessment of the program is vital; if they don’t like it, they won’t use it. Patient-reported outcome tools are valuable in this regard as are tools that assess depression, an underrecognized issue for people with sickle cell disease.

A patient registry provides valuable insight on the impact of network expansion. Registry data are useful both to improvement in patient care and program refunding. Key items must be identified and agreed upon prior to registry implementation. Important issues include the specific data to be captured and by whom, security and control of data access, patient consent, data maintenance and cleaning as well as the data collection platform.

Financial assessment including the cost to run the program (cost to run each spoke), impact of cost, hospitalizations/rehospitalizations, and transfusion costs is paramount. Sickle cell disease care programs do not make money for institutions. However, cost reduction and cost avoidance can positively affect the institutional balance sheet. Clear documentation of baseline and subsequent program costs is essential.

Conclusions
Programs to expand care to needy patients with sickle cell disease are born from compassion but can die from lack of data to support that compassion. Unfortunately, the human value of an interaction between a caring physician and a patient cannot be measured. Therefore, scrupulous record keeping is needed for other components of that interaction that can be quantified. Patience and persistence along with clarity and focus in planning are essential to program development. Not enough can be said on the value of operational dexterity and flexibility. Final programs never reflect drawing board representations. Lastly, continuous engagement with patients and families, payers, as well as local and federal government allows continuous reassessment which contributes to long-term program success.

References


56. University of New Mexico School of Medicine. Project ECHO. Available at: https://echo.unm.edu/about-echo/model/. Accessed May 12, 2019.


