Sickle Cell Disease, 2015

A Patient Advocate's Perspective

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Background

Improved health care for children with sickle cell disease has increased the likelihood of survival to young adulthood for those born in the U.S. with this inherited rare disease. However, despite improving pediatric clinical outcomes, challenges remain: Children and adults have limited access to high-quality health care; overall survival remains decreased, pain management is supportive without any disease-specific pain interventions, transition to adult care is poorly organized, and development of disease-modifying therapies and universally available cures remains limited. Federal organizations, healthcare systems, scientific and medical societies, patient advocacy organizations, and other prominent stakeholders in the sickle cell disease community have offered solutions to these problems but policies to institute change have not emerged. Many have suggested that if the sickle cell disease community would unite to focus on advancing a common agenda, one "voice," then meaningful change would result.[1,2] The overall concerns of these stakeholders should not be too disparate. One would imagine that all could agree to focus on the following goals:

1. increasing access to high-quality patient care;
2. developing drugs and therapies to increase survival and improve quality of life; and
3. finding cures for sickle cell disease that are relatively accessible, safe, and with limited long-term complications.

Partnerships must develop among stakeholders in the sickle cell community, and a unified voice must emerge that resoundingly reflects the concerns of the patients and their families. Focusing on the goal of increasing access to high-quality patient care, this editorial explores challenges and solutions from the patient advocacy organization perspective.

The concept of "access to care" is broad and complex for those living with chronic illness. Many Americans with sickle cell disease have socioeconomic challenges that include living in households with low family incomes, residing in geographic locations with few sickle cell disease healthcare experts, attaining and maintaining health insurance, and trying to lead productive lives with a chronic medical condition. In addition, racial and cultural differences between the patient and members of the healthcare community may exist that impede effective communication and therapeutic relationships. Furthermore, the stigma associated with living with sickle cell disease further isolates children and adults. For these and other reasons, children and adults with sickle cell disease may benefit from services provided by sickle cell disease community-based organizations.

Individuals living with sickle cell disease are likely to define high-quality health care similar to the general population. This definition would include the idea of having the best health care available to you when you need it and having it delivered in a way that provides the best outcomes. IOM embodies this concept in their six fundamentals of high-quality health care: safe, timely, patient-centered, effective, efficient, and equitable. The body of work presented in this special supplement addresses many of these fundamentals. The following provides more-specific elements on how children and adults with sickle cell disease should be provided access to high-quality health care:

1. Access to high-quality care should be universal across a wide variety of primary care and specialty care settings.
2. Systems to monitor quality indicators of care should be in place across care settings.
3. Efforts to improve clinical care for people with sickle cell disease should address care for adults and children.
4. Disease self-management skills should be an element of high-quality health care across the life span.
5. Children and adults with sickle cell disease and their families should have accessible resources to identify

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high-quality health care and sickle cell disease–related advocacy initiatives both nationally and within their communities.
6. Resources to better identify and access sickle cell disease community-based organizations and advocacy organizations should be developed.
7. The sickle cell disease community should collect and have access to data that inform healthcare advocacy initiatives for research, health care, education, and awareness.
8. Funding and policies to support and sustain high-quality clinical care for children and adults with sickle cell disease should increase.
9. Funding to support innovative science and clinical research trial participation should be increased.

Access to High-Quality Care

Solutions to broader access must address outpatient, inpatient, and acute/emergent care. Pain management is challenging, particularly when emergent care settings are used. Day hospitals and alternative urgent care may provide pain management in a setting conducive to prompt treatment and extended stays until pain can be managed in an outpatient setting. These day hospitals or urgent care units may combine resources with other outpatient settings such as transfusion or infusion units designed to provide care for oncology patients. Despite the setting of initial pain management, connecting patients to their medical home team shortly after acute illness visits should lead to better outcomes. Putting teams in place will ensure that prescriptions have been filled and treatment plans are being followed. Ensuring that patients have follow-up appointments within 24 hours and analgesics in hand prior to discharge or shortly after discharge may increase the likelihood that their outpatient pain management will be successful, hopefully leading to reduced readmissions. This type of care coordination is necessary for patients with sickle cell disease across the life span. Children and adults have screening procedures for stroke risk and pulmonary hypertension that rely on ancillary services. A community-based organization involved in care coordination would be best suited to understand the medical and non-medical needs of the patient and to identify resources in local communities.

Systems to Monitor Quality Indicators of Care

Safe, timely care is central to pain management across all settings but particularly for emergent care. Emergency medicine healthcare teams must be involved in developing clinical pathways that emphasize pain relief and implementing quality indicators such as timing to first analgesic. Inpatient units should pay close attention to the side effects of analgesics and the development of acute chest syndrome. These processes should be developed across care settings and include febrile illness and neurologic symptom management. Once developed, a systematic tracking method should allow data sharing to support quality improvement initiatives. Healthcare systems should share information with consumers to allow patients to make informed decisions about where they receive their health care. This type of monitoring could be built within the medical home and care network structure, but their outcomes reporting should include community-based organizations and patient advocacy organizations.

Because of the subjective nature of pain and the need to include patients in its assessment, tension between patients and their healthcare teams develops when there is discrepancy between a patient's desired pain management and the plan of their healthcare team. Patients and their families should be involved in pain management decisions when appropriate. A patient's bill of rights has been helpful in cases where distrust has developed between patient and care teams. However, patients should be equally attentive to respectful, positive communication and interaction with health teams. Restoring trust is typically more difficult, but should be considered the priority when trust dissipates. Healthcare providers in all settings should be educated on cultural competency and conflict resolution strategies with a goal of rebuilding trust. Patient and parent support groups should be encouraged to include healthcare providers in their discussions of ways to improve hospital experiences.

Care for Adults and Children

Historically, many medical advances in sickle cell disease research have addressed outcomes more frequently in children than adults. Infection and stroke prevention has saved and improved lives of American children with sickle cell disease. Compare these advances in pediatrics to those in adult-focused care. The greatest medical advance in adult-focused care has been related to one disease-modifying agent, hydroxyurea. However, pain and acute chest syndrome remain the top causes of morbidity and mortality in adults with sickle cell disease. Given the continued early mortality in adulthood and the low percentage of adults receiving long-term therapies, new models of care to address access to high-quality care in adults need to be more expedient.

Disease Self-Management Skills

Patients should be empowered with information that allows them to better identify concerning symptoms and improve adherence with medication. No patient or parent of a child with sickle cell disease should leave a healthcare setting without a better understanding of how to care for themselves or their child. Patient education should include resources that go beyond direct communication with the medical team. Written health education materials and computer and web-based resources enrich patient education. Community-based organizations with expertise in sickle cell disease can make a meaningful difference by providing information to patients and their families.

Accessible Resources for Advocacy Initiatives

Community-based organizations that provide useful resources and services that decrease barriers to care are critical. However, resources that detail information on types of provided services, organized in a user-friendly, readily accessible fashion, are limited. Improved disease awareness initiatives should communicate these concerns and foster clinical care improvements, novel disease-modifying therapies, and cures in a manner that is most meaningful to patients. Clinical research designs that support trials that inform probabilities of health outcomes based on individual patient factors are needed. Clinical trial endpoints should address complications that are important to patients, particularly those that reflect quality of life. Disease-specific quality of life assessment tools are close to completion and should be incorporated into clinical care and clinical research.

Without a disease surveillance program, tracking access to high-quality care and clinical outcomes is extremely limited. However, the use of large healthcare information databases to estimate utilization of healthcare services by children and adults with sickle cell disease could be a solution to better understand variation in patterns of care. Transition of care from home to acute care settings as well from pediatric- to adult-focused care is a major barrier to continuous, high-quality health care across the life span. A poorly resourced healthcare workforce expert in the care of patients with sickle cell disease is a frequently referenced problem in the sickle cell disease community. When patients are coming from home to emergency departments for urgent/emergent health issues, they are often reluctant as they worry about their ability to get culturally competent, compassionate, safe, and high-quality care in these settings. Ultimately, they are concerned that they are not receiving care from a healthcare provider knowledgeable about their sickle cell disease. Ensuring effective communication between their primary healthcare team and emergency care teams about their accurate health information may resolve some of these issues. For those that do not have a primary care team or a medical home, systems should be developed to connect them to one. Partnerships with healthcare systems and community-based organizations should be developed to address this issue.

Even when adult care–focused providers are available, transition from pediatric- to adult-focused health care is challenging. The role of the parent and pediatric team is critical. Limiting their participation and control over the process is difficult and problematic. Health system issues are challenging to navigate even for those without chronic illness and abundant resources. Understanding the concerns of patients and their families, healthcare providers, and leaders in health systems communities should be instrumental in finding solutions. Health information technology is an important resource that could be incorporated into transition programs from both the provider and patient perspectives. Using electronic health records to share information between pediatric and adult care providers and allowing both providers to track outcome measures of successful transition should be considered.

Access to Community-Based Organizations

Enlisting the services of community-based organizations could clearly improve access to health care that may be limited because of a patient's poor understanding regarding the need for a variety of healthcare providers, difficulties with transportation to care centers, problems with maintaining health insurance and navigating complex healthcare systems, and difficulty in communicating with healthcare teams and poor adherence to medication. Understanding the strengths of individual community-based organizations and focusing on methods to improve resources, develop new programs, and sustain positive outcomes through continued funding is critical to establishing quality measures for all community-based organizations. For example, if relationships between hospitals and community-based organizations are fostered at both the pediatric and adult levels, then transitions of care may be improved by using the local community-based organization as a backbone to reinforce patient education, facilitate care coordination, and address barriers to successful school and work performance at the individual level. Behavioral health problems in the sickle cell disease community are under-recognized and subsequently poorly treated. Bringing assessment tools on quality of life into the sickle cell community may

be a method of screening for high-risk features for behavioral health issues. Increasing awareness of behavioral health issues in the community and identifying support services through community-based organizations may decrease the stigma associated with behavioral health needs.

Access to Data

Owing to variation in services provided by states for children with sickle cell disease identified through newborn screening, the opportunity to track outcomes is not consistent within and throughout the states. The opportunities for newborn screening follow-up are numerous but are plagued with important issues such as health information privacy, inconsistent coding practices, lack of data storage, and poor standardization of needed data elements. Programs for disease surveillance, disease outcomes tracking, and monitoring access to high-quality health care would be a worthwhile endeavor and a natural progression of state newborn screening programs. With participation of community-based organizations and healthcare systems, the opportunity to track children and adults with sickle cell disease would be bolstered. This would allow the collection and analysis of important data to better inform health policy for those with sickle cell disease.

Therapies directed at improving quality of life, particularly for adults with sickle cell disease, are needed. Cures for both children and adults with all genotypes of sickle cell disease are long overdue. To improve health care for sickle cell disease, access to high-quality health care throughout the life span; therapies to improve quality of life and cure sickle cell disease; education for consumers or patients, families, and healthcare providers; and awareness in the communities must be the focus of such efforts. However, in this context, issues of disparities of care, culturally sensitive community engagement, healthcare system complexities, disease stigma, behavioral health issues, ever-changing health policies, and third-party payer regulations must be addressed to develop an advocacy platform that serves all.

High-Quality Clinical Care for Children and Adults

Access to resources is greatly limited. This has fragmented the sickle cell disease community. Researchers and clinical care centers compete for limited federal and foundation dollars. Patient advocacy and community-based organizations compete for philanthropic dollars and private industry sponsorship. Many children and adults with sickle cell disease live below the poverty line and require additional support to access and maintain access to health care. In this environment, it is paramount to have consistent and sustainable funding to support excellence in clinical care, research, and care coordination services. Legislation is poised to secure funding, yet despite all efforts, the H.R.1807 Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act 2015 has not been reauthorized. Sickle cell disease should be excluded under the Readmissions Reduction Act so that hospitals will be not be penalized for the care of patients with sickle cell disease where return to hospitals for management of febrile illness and unpredictable pain may be unavoidable. Policies related to chronic opioid use and medical marijuana could greatly impact patients living with sickle cell disease. Patients and patient advocacy organizations need to be ready to address these issues proactively before further legislation negatively affects access.

Innovative Science and Clinical Research Trials

Although advances in medicine have led to improved outcomes for some, the cure for sickle cell disease and the sole disease-modifying drug are not available, accessible, or effective for all patients. To develop new cures and disease-modifying agents, innovative basic science and clinical research must be initiated and funded. In addition to a diminishing healthcare workforce available to provide high-quality clinical care for adults with sickle cell disease, many believe that young scientists and clinical investigators are losing interest in hematology. Programs developed to improve resources and access to mentors in science, technology, engineering, and math areas could resolve this issue. On the patient side, increased clinical trial participation is needed. However, reasons for slow, limited clinical trial participant recruitment are unknown. Though some suggest that mistrust plays a key role,[3] poor resources are available to support clinical trial participants who may have limited socioeconomic resources and whose disease or treatments may limit their ability to fully participate. Further investigation is needed to appropriately address the barriers to clinical trial participation from both the view of the patient and that of the healthcare system and private industry. Concentrating on developing therapies for outcomes that are meaningful to the sickle cell disease community is paramount.

Conclusions

Ultimately, many of the concerns of the sickle cell disease community could be resolved by developing an

information technology–based system for connecting patients to the healthcare community, thus allowing them to identify and access

1. healthcare providers in their community who provide high-quality care for patients with sickle cell disease;
2. clinical research projects;
3. advocacy and awareness projects; and
4. psychosocial support services.

This initiative is best supported by a patient advocacy organization that could secure and maintain funding as well as stakeholder commitments. Resoundingly, the stakeholders within the sickle cell disease community must focus on access to high-quality health care. However, the interests of children and adults with sickle cell disease must be prioritized within this context. For although a unified message is an important concept to carry a rare disease–focused agenda forward in terms of research funding and health policy initiatives, if the message does not reflect the concerns and needs of patients, then that "one voice" will be forever divided.

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