

**SICKLE CELL DISEASE  
FORUM ENGAGING  
THE COMMUNITY:  
DEVELOPING SOLUTIONS**

NATIONAL INSTITUTES OF HEALTH CAMPUS  
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## WELCOME AND OPENING REMARKS

**Lenora Johnson, Dr.P.H.; Director; Office of Science Policy, Engagement, Education and Communications (OSPEEC); National Heart, Lung, and Blood Institute (NHLBI)**

Dr. Johnson welcomed presenters and audience members to the Sickle Cell Disease (SCD) Forum.

### SCD RESEARCH: AN NHLBI PRIORITY

**Gary H. Gibbons, M.D., Director, NHLBI**

Dr. Gibbons welcomed all participants to the SCD Forum, which he said would provide important information about SCD research and management. He noted that the Forum would also offer an opportunity to chart the future for improvements in care and funding strategies.

Dr. Gibbons emphasized community and commitment. He said that NHLBI plans to engage its entire community of partners—including patients, families, providers, researchers, professional societies, academic institutions, and health systems—to enhance care for patients living with SCD. Dr. Gibbons indicated that SCD research is a top scientific and research priority for NHLBI that could transform lives and NHLBI is committed to improving the health and quality of life of patients with SCD. He believes that a cure is on the horizon, and finding a cure is NHLBI's goal.

### PROGRESS IN SCD RESEARCH AT NIH

**Francis S. Collins, M.D., Ph.D., Director, National Institutes of Health (NIH)**

Dr. Collins highlighted progress made at NIH in several areas:

- Drug development. The National Center for Advancing Translational Sciences at NIH helped advance the development of an SCD drug that reverses the sickling of sickle hemoglobin.
- Genomics. NIH-supported research showed that suppressing the BCL11A protein leads to the production of more fetal hemoglobin.
- Gene therapy. Researchers have inserted a normal copy of a gene into stem cells that form blood cells in the bone marrow. After this gene therapy, a patient had no pain and did not need blood transfusions for several months.
- DNA editing. The ability to edit DNA code using the clustered regularly interspaced short palindromic repeats (CRISPR-Cas) system might be used to fix the SCD gene mutation. Cells with the healthy gene could then be transplanted into bone marrow to form healthy red blood cells.
- Bone marrow transplantation. This procedure has cured some patients with SCD.

## GENERATING HOPE FOR SCD

**Wayne A. I. Frederick, M.D., M.B.A., President, Howard University**

Dr. Frederick was diagnosed with SCD as a newborn in Trinidad and Tobago. His parents were told that he would not live past age 8. As a boy, Dr. Frederick was hospitalized four to six times each year. He eventually attended Howard University, where he found a family of other patients and advocates. The support from this community and the SCD Center at Howard gave Dr. Frederick hope that he could control his disease.

After obtaining his medical degree, Dr. Frederick applied to a residency program that requested a doctor's note verifying that he was healthy enough to complete the program. Dr. Frederick withdrew his application from the program in response to this request and completed his surgical residency elsewhere. During his residency, he hid his many pain crises from most of his colleagues.

Dr. Frederick continues to be hopeful. The SCD field has made tremendous strides, and the research at NHLBI is headed in the right direction. Giving patients hope that they can lead a normal life is key to researchers' efforts.

## BUILDING AWARENESS FOR SCD

**Wanda Whitten-Shurney, M.D., Director, Newborn Sickle Cell Screening Program, Children's Hospital of Michigan**

June 19 was not only World Sickle Cell Day but also the date when a landmark article was published in the New England Journal of Medicine showing that penicillin can prevent infections in children with SCD.

Only one disease-modifying medication, hydroxyurea, is available for SCD. Patients are afraid to take this medicine, and doctors are afraid to prescribe it. Awareness of SCD trait status is another unmet need. Young adults need to find out their SCD trait status so that they can make informed decisions about childbearing and know the challenges they might face.

Ignorance is rampant. Adults with SCD frequently visit the emergency room knowing the pain medications and doses they need. Doctors often believe, however, that these patients are trying to get painkillers for reasons not associated with SCD. These doctors need to realize that these patients are truly in pain, that the pain is related to SCD, and that the patients require treatment.

## PANEL 1: SCD RESEARCH: PAST, PRESENT, AND FUTURE

### PROGRESS IN SCD RESEARCH

**Clarice Wills Reid, M.D., Former Director, Division of Blood Diseases and Resources, NHLBI**

Some of the first milestones in SCD were:

- 1910: Dr. James B. Herrick, a Chicago physician, first described SCD.
- 1949: Dr. Linus Pauling, a well-known chemist, described SCD as a genetic disease.
- 1957: Dr. Vernon Ingram, a biologist at Cambridge University, identified the amino acid changes in the hemoglobin of people with SCD.

Dr. Reid described the 1970s as the age of enlightenment for SCD, when scientists' understanding of SCD exploded. The 1980s were the renaissance era for SCD research. Biologists, geneticists, and physician scientists identified the globin gene and its role in the disease, which led to the development of hydroxyurea. The 1990s were the age of therapeutic triumph. Science produced a trove of information on how to turn on the globin gene, and experts hoped to develop many new therapies. Dr. Reid believes that we have now entered the age of cure.

### ROLE OF NIH IN PREVENTING AND REDUCING STROKES IN CHILDREN WITH SCD

**Harvey Luksenburg, M.D., Acting Branch Chief, Blood Diseases Branch, Division of Blood Diseases and Resources, NHLBI**

Stroke is a leading cause of death and disability in children and adults with SCD. Transcranial Doppler ultrasound led to a revolution in preventing strokes in children with SCD. This technology can identify children with a high risk of stroke.

The NHLBI-sponsored Stroke Prevention Trial in Sickle Cell Anemia (STOP) found that children with SCD were much less likely to have a stroke if they had regular blood transfusions. These findings changed clinical practice. The STOP II trial showed that transfusions should continue indefinitely in children with SCD and a high risk of stroke.

Questions for further research are:

- Can hydroxyurea (instead of transfusions) prevent strokes in children?
- What are the best ways to predict and treat stroke in adults?

### SCD RESEARCH AT NHLBI: STRATEGIES FOR THE NEAR FUTURE

**W. Keith Hoots, M.D., Director, Division of Blood Diseases and Resources, NHLBI**

NHLBI will announce soon a new funding opportunity for medical centers that treat SCD in patients aged 15 to 45 years. The award will fund efforts to measure the effectiveness of various strategies aimed at improving access to care and outcomes of care. Another new NHLBI program, the Global SCD Initiative, will enhance the research infrastructure in Sub-Saharan Africa. NHLBI continues funding research to develop new drugs and other treatments for SCD. A new NHLBI challenge competition funding opportunity involves teams of undergraduate students in the United States educating communities about SCD.



## DISCUSSION

Participants offered the following comments during the discussion:

- NHLBI is completing the documents needed to get Food and Drug Administration (FDA) approval for hydroxyurea treatment in children.
- Incentives and reimbursement for transportation costs can increase enrollment in clinical trials.
- Science has already found some treatments that seem to work for SCD, but there continues to be a gap between what we know and what we do.
- Research needs to address the long-term physical and mental consequences of SCD.
- Experts in many different specialties need to work with patients to find a cure for SCD.

## PANEL 2: MANAGING SCD PAIN

### MANAGING SCD PAIN

**Wally R. Smith, M.D., Florence Neal Cooper Smith Professor of SCD, Virginia Commonwealth University Medical Center**

The factors that affect responses to pain and whether patients seek treatment or stay home include gender, readiness to get treatment, health status, and psychosocial factors (such as stress or depression).

The Pain in Sickle Cell Epidemiology Study (PiSCES) showed that 30 percent of patients with SCD have pain at least 30 percent of the time, and another one-third have pain every day. Patients manage about three-quarters of their pain crisis days at home. Therefore, the pain that doctors are told about at the hospital represents only a small fraction of the pain that patients actually have. Fatigue might be as important as pain to patients' quality of life. Researchers have difficulty differentiating fatigue and pain, and they may influence each other.

### NEW TREATMENTS FOR SCD PAIN WITH METHODS TO MEASURE PAIN

**Kalpna Gupta, Ph.D., Professor of Medicine, Division of Hematology, Oncology and Transplantation, University of Minnesota**

Many features of SCD seem to cause pain, including decreases in oxygen levels in the blood, nerve defects, and inflammation. Even after the body heals, the factors causing pain might remain in the in the spinal cord and continue to cause pain.

Dr. Gupta is developing a way to measure pain in mice with SCD based on their facial expressions in response to pain. Her findings might lead to smartphone applications that can transmit photographs of patients' facial expressions to their doctors to get rapid pain treatment without leaving home.

Dr. Gupta is also studying the use of curcumin (a substance in turmeric) and coenzyme Q10 (a substance in the body that is similar to a vitamin) to reduce pain in mice with SCD. The results show that changing the diet or giving patients supplements might reduce pain. Another promising pain treatment in SCD is acupuncture. Studies in Dr. Gupta's laboratory also show that cannabinoids (a substance in marijuana) can reduce SCD pain in mice.

### MANAGING SCD PAIN WITH OPIOIDS AND TRANSCRANIAL MAGNETIC STIMULATION

**Ricardo A. Cruciani, M.D., Ph.D., Director, Neurology; Director, Center for Comprehensive Pain Management and Palliative Care; Director, Laboratory for Neuromodulation and Plasticity at Capital Institute for Neurosciences, Capital Health Regional Medical Center**

Whether opioids are a good treatment for pain in patients with SCD is controversial. Because opioids seem to have both positive and negative effects on SCD symptoms, doctors should consider treating the side effects of opioids when they use these drugs. The risks and benefits of long-term opioid use in chronic pain have not been established. Some patients, however, do benefit from long-term opioid therapy. The side effects of opioid use revealed in animals need to be studied in humans, and the risk of accidental overdose must be lowered.

Researchers have shown that transcranial magnetic stimulation of the brain can significantly reduce pain in some patients.

## MANAGING SCD PAIN: THE PATIENT-CLINICIAN RELATIONSHIP

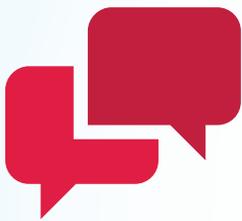
**Carlton Haywood, Jr., Ph.D., M.A., Assistant Professor, Division of Hematology; Core Faculty, Johns Hopkins Berman Institute of Bioethics; Core Faculty, Welch Center for Prevention, Epidemiology, and Clinical Research; Johns Hopkins School of Medicine**

People with SCD in different studies and different countries tend to describe their pain experiences in remarkably similar ways. Many report that health care providers treat them with mistrust and do not believe that they are in as much pain as they say they are. Providers sometimes stigmatize these patients as addicted or drug seeking. Some providers do not seem to want to let these patients participate in decisions about their own care; some show no sympathy or compassion.

Negative clinician attitudes about people with SCD and poor provider knowledge are two of the most important barriers to appropriate pain management for people with SCD. However, studies have found that some interventions can improve pain management in people with SCD. These interventions need to be extended to the places where SCD patients get care.

Future directions in this line of research include answering the following questions:

- What model(s) of care leads to the best outcomes for patients with SCD?
- What educational approaches improve clinician attitudes the most toward people with SCD?
- How should new knowledge and understanding about the biology of SCD pain be incorporated into education on pain self-management for affected individuals?



### DISCUSSION

Participants offered the following comments during the discussion:

- Different people express pain differently. Dr. Gupta's technology can be used to measure even subtle changes in facial expression.
- Patients who have lived with SCD from birth tend to develop coping mechanisms and might express pain differently than people without the disease. Dr. Gupta plans to assess facial expressions in people with SCD pain before treatment for a baseline comparison.
- Chronic fatigue in SCD is closely linked to chronic pain.
- In SCD, patient and provider assessments of pain do not correlate well, which contributes to communication problems.
- More evidence is needed on the use of different forms of cannabis in medicine.

## PANEL 3: INNOVATIVE CARE MODELS

### SCD: INNOVATIVE MODELS OF CARE

**Sophie Lanzkron, M.D., M.H.S., Associate Professor of Medicine and Oncology, Johns Hopkins School of Medicine**

While SCD patients typically get acute care in emergency rooms, which costs \$1.5 million for every 100 patients, the Johns Hopkins Sickle Cell Infusion Clinic saves five treatment slots every day for acute care visits. On average, patients spend about five hours in the clinic, and 85 percent of those with vaso-occlusive crisis go home after treatment. The hospital admission rate for vaso-occlusive crisis throughout Maryland dropped from 50 percent to 20 percent since the clinic began operating in 2008.

Several factors explain the success of the Johns Hopkins Infusion Clinic. The clinic treats pain quickly and aggressively, and its caregivers are dedicated to delivering high-quality care to people with SCD. In addition to pain management, the clinic offers social work and psychiatric services.

## COMMUNITY-BASED SUPPORT FOR SCD CARE

**Lewis Hsu, M.D., Ph.D., Director, Pediatric Sickle Cell Program; Professor of Pediatrics, University of Illinois at Chicago**

Dr. Hsu proposed making community-based support a national priority for SCD care. Community health workers and patient navigators are effective in other chronic diseases. They help patients find their way through the hospital or clinic, figure out who to call for help, and know how to navigate the health care system. They are particularly valuable for underserved communities.

The idea of using community health workers in SCD is very new, and many research questions need to be answered. For example, research needs to show which components of a community health worker program make a difference for patients with SCD. Studies also need to measure the outcomes of such programs.

## AN EMERGENCY PHYSICIAN'S EXPERIENCE WITH SCD

**Jeffrey Glassberg, M.D., Assistant Professor of Emergency Medicine and of Medicine, Hematology, and Medical Oncology, Icahn School of Medicine, Mount Sinai; Director, Mount Sinai Comprehensive Sickle Cell Program**

As an emergency physician, Dr. Glassberg is exposed to every discipline within medicine. SCD is a disease of the blood, which flows to every organ. Therefore, SCD is a disease of every organ.

Dr. Glassberg is leading a study of inhaled steroids for people with SCD who do not have asthma. He is also comparing individualized and weight-based pain dosing for managing SCD pain in the emergency room.

At Mount Sinai, patients can move quickly from initial evaluation in the emergency room to a quieter place in the facility. They also can obtain pain management without being admitted to the hospital, an approach patients appreciate.



## DISCUSSION

Participants offered the following comments during the discussion:

- The infusion center models that were described are unlikely to make money, so hospital administrators might not be willing to pay for them.
- One way to show hospital administrators the value of infusion clinics for SCD care is to point out that such programs lower hospital admission rates.
- Most communities struggle to engage providers who are not involved in SCD care. It can be difficult to engage primary care clinicians and community-based hematologists, for example.
- One deterrent for patients going to the emergency room is having to explain what they are experiencing and which medication works best for them. A solution is a voluntary database of medical records for all patients with SCD.
- Patients with SCD should carry their records with them whenever they go to the hospital or see a doctor. A single page can provide the patient's contact information, insurance details, drugs, and recommended pain treatment from a doctor.

## UPDATE FROM THE AMERICAN SOCIETY OF HEMATOLOGY (ASH)

**David A. Williams, M.D., President, ASH**

ASH represents 15,000 practicing hematologists, scientists, and trainees around the world who are committed to studying the treatment of blood diseases. ASH has taken several steps to ensure that patients receive state-of-the-art care and attention, including:

- Released priorities for SCD and sickle cell trait.
- Encouraged research focused on SCD in the 2015 ASH Agenda for Hematology Research.
- Held a congressional briefing on SCD in 2014 cohosted by ASH and the Sickle Cell Disease Association of America (SCDAA).
- Made resources available for clinicians, including quick-reference guides on SCD.
- Developed resource web pages for patients, including the ASH Find a Hematologist page.

ASH hosted an SCD summit to identify the highest-priority actions to advance outcomes for people with SCD and to map a plan to advance those actions. In the next few months, ASH will refine and prioritize the issues identified at the summit and determine stakeholders who could help advance these efforts. The society plans to launch an SCD web page that will list the society's tools and resources along with links to federal agencies, philanthropic programs, and funding opportunities for research. ASH will also host a webinar, likely the first of several, for health care providers on how to best use hydroxyurea to manage SCD.



### DISCUSSION

Participants offered the following comments during the discussion:

- ASH should include FDA's SCD patient representatives in its future SCD planning.
- The ASH SCD summit identified a major need to increase the number of adult hematologists trained in SCD treatment, and ASH will redouble its efforts to educate community physicians.
- Loan repayment programs are needed for the training of SCD providers.

## PANEL 4: SCD TRANSITIONS

### TRANSITION OF CARE IN SCD

**Julie Kanter-Washko, M.D., Director of SCD Research and Assistant Professor of Pediatric Hematology-Oncology, Medical University of South Carolina**

Patients often have huge spikes in pain as they get older and their disease complications worsen. The treatment of the disease also changes as the patient moves from the pediatric to the adult care environment. The adult health care system is very different from the pediatric system. Whereas the pediatric health care system is nurturing and protective, for example, the adult system is focused on keeping patients informed. The pediatric system centers on parents, whereas the adult system centers on patients. The pediatric system is coordinated and centralized, and the adult system is fragmented and has few SCD providers. Questions that need to be answered about successful transitions for patients from pediatric to adult care include:

- Who defines success?
- What is success?
- How can researchers measure success in transitions?

## INNOVATIVE MODEL OF CARE FOR SCD TRANSITIONS

### **Brooke Allemang, M.S.W., R.S.W., Transition Navigator, Hospital for Sick Children and Toronto General Hospital**

Ms. Allemang is a health transition navigator who works in a pilot program at both a pediatric hospital and an adult hospital. The program's goal is to ensure that all patients leave the pediatric environment with all of the knowledge, skills, and tools they need to use adult services appropriately and advocate for themselves.

To prepare young people for the transition, Ms. Allemang introduces the transition concept during regular clinic visits. She shares a policy with patients and parents on self-management, and she reviews what to expect as they leave the pediatric system and enter the adult system. Ms. Allemang uses a survey to assess each patient's level of understanding and knowledge of SCD, self-management ability, and readiness for adult care. She also helps patients set transition goals and track how they are doing in achieving these goals.

In the transition clinic for patients aged 17–18 years, each young person and his or her family consults with an adult hematologist in the presence of a pediatric hematologist. The adult hematologist teaches the patient about the adult system and helps the patient become comfortable with adult providers. Each patient creates a "My Health Passport," a wallet-size medical history summary with useful information the patient can refer to when seeing a new provider. The program continues to support patients aged 18–21 after the transition to the adult clinic.

## CHRONIC PAIN IN SCD

### **Christopher Patrick Carroll, M.D., Director of Psychiatric Services, Sickle Cell Center for Adults, Johns Hopkins Hospital**

Approaches to acute pain and chronic pain management tend to be different. Acute pain is related to a clearly identifiable injury, and its management focuses on reducing severity while letting the main cause of the pain heal. Chronic pain persists for a long time after the precipitating event has ended. Interventions for chronic pain tend to have modest effects, at best. The goals of chronic pain management are to maximize function and quality of life while reducing pain intensity.

Transition issues in patients with SCD who leave pediatric clinics and start receiving their care in adult clinics are magnified by the range of challenges that SCD presents. At this stage, patients shift from intermittent episodes of acute pain to more severe pain in between crises, sometimes experiencing pain daily. Pain treatment approaches might need to change during the transition phase for patients to focus more on functioning. Patients need help preparing for this transition earlier, prior to shifting fully into the adult care system.



## DISCUSSION

Participants offered the following comments during the discussion:

- During the transition to adult care, young adults need to consider the types of SCD care available at the colleges they may wish to attend.
- In the Hopkins adult clinic, providers focus on behaviors that interfere with patient functioning, such as not coming in for regular transfusions. The providers create a multidisciplinary plan to reduce these challenging behaviors.
- Ms. Allemang always asks patients about the best way to communicate with them about their SCD, and patients often prefer text messages to telephone calls.
- Age 18 may not be an ideal time to make the transition from pediatric to adult care because these patients could also be leaving home and experiencing many other changes.
- Lack of sleep seems to be related to pain and depression. Treating only one of these problems and not the others is unlikely to work.
- More physicians, especially those who only see a few patients with SCD, need to learn how to prescribe hydroxyurea.
- Home nurses can assess patients and help them make the best use of the resources available to them while at home. However, patients need to be evaluated in the hospital during severe acute episodes.

## PANEL 5: IMPORTANCE OF CLINICAL TRIALS

### THE IMPORTANCE OF CLINICAL TRIALS IN SCD

#### **Swee Lay Thein, M.D., D.Sc., NHLBI (Panel 5 Chair)**

Survival rates improved in children with SCD between 1979 and 2006, and up to 98 percent of newborns with SCD survive at least until adulthood in developed countries. The reasons for these improvements include the expansion of newborn screening, use of penicillin to prevent infections, use of blood transfusions to prevent stroke in children, and hydroxyurea treatment.

Patients with SCD need to see improvements in the current disease-management strategy, which focuses on limiting damage by recognizing problems early and treating them. They need better supportive and general medical care, more treatment options, and better ways to predict disease severity and disease course.

Clinical trials reduce the risk of bias in generating evidence. To make the advances that patients need, patient participation in clinical trials is critical.

### OPEN CLINICAL TRIALS FOR SCD

#### **Kim Smith-Whitley, M.D., Director, Comprehensive Sickle Cell Center, Children's Hospital of Philadelphia; Chief Medical Officer, SCDA**

Clinical research focuses on finding better treatment, detection, and prevention approaches for a disease. Investigators design their trials to show objectively whether a new medication or treatment is superior to those used currently.

Randomization is the process that researchers use to decide whether each participant will be treated with a drug or a placebo (sugar pill). Otherwise, researchers might assign their sickest patients to the real drug that they think will work or only assign the patients who are healthiest to a drug that they think will not work. Randomization eliminates bias so that the study can show whether the drug does or does not work.

FDA has requirements for how studies measure outcomes and show statistical differences between a drug and a placebo. The endpoint measured in SCD trials is often focused on preventing or treating pain. Some drugs currently in development change the course of pain episodes. The objectives of some studies also include trying to reduce complications of SCD, like ulcers and fatigue.

The public can search <https://ClinicalTrials.gov> to find ongoing studies related to SCD. The site provides information on each study, such as the locations of study sites and the drugs being tested.

### IMPORTANCE OF CLINICAL TRIALS IN ESTABLISHING CURES FOR SCD

#### **John Tisdale, M.D., NHLBI, NIH**

Survival rates for childhood cancer have improved substantially over the last several decades because children with cancer participate in clinical trials and clinical trial participation leads to cures. However, participation rates decrease with age. Unlike children with cancer who die without treatment, children with SCD live into adulthood. Therefore, people become complacent about encouraging parents to enroll their children in clinical trials. Although deaths in children with SCD have virtually disappeared, in 2006 the mean age of death in adults with SCD was only 39. This disease is still terrible to live with and it compromises quality of life.

A cure for SCD is bone marrow transplantation. This procedure replaces the "seeds" of blood cells in bone marrow so that they can make healthy red blood cells. Young children with SCD, a risk of stroke, and a relative whose blood cell markers match the patient's markers should consider a bone marrow transplant. One reason why transplants are not used more often is that patients must be treated with high doses of chemotherapy and radiation. Adults with SCD, who already have substantial organ damage, cannot withstand this toxic treatment. However, bone marrow transplantation can be done with lower doses of chemotherapy and radiation in adults with SCD to prevent rejection of the transplanted cells. Unfortunately, access to the procedure is still limited because most patients do not have a family member with matching blood antibodies who could donate bone marrow cells.

Another way to offer adults with SCD a bone marrow transplant is to use bone marrow stem cells from the patient. Researchers add copies of healthy genes to the stem cells before transplanting them back into the patient. All of the cells that grow in the patient's bone marrow should then produce normal hemoglobin. This strategy seems promising, but more research is needed on its long-term effects.



## DISCUSSION

Participants offered the following comments during the discussion:

- New statistical techniques let investigators design more efficient clinical trials that can accomplish many goals at once, but these trials do not fit NIH funding criteria or FDA requirements to approve a new treatment.
- New ways are needed to spread awareness of clinical trials using community-based and patient advocacy organizations. SCDAA's Get Connected program can also help.
- Patients living with SCD should have rapid access to therapies shown to be effective in clinical trials.
- Many patients with SCD are not interested in participating in a clinical trial because they have so much trouble getting basic care at their local hospital. In addition, enrolling in a clinical trial does not necessarily benefit them.
- Many clinical trials provide excellent standard care, and this care might be better than the care that patients typically receive.
- Clinical trials are the only way to develop a drug that reduces pain in people with SCD.
- Consent forms for clinical trials are long and hard to understand.
- Physicians need to tell patients about the potential benefits and risks of a bone marrow transplant, and patients should use this information to decide what to do.

# APPENDIX 1: SUMMARY OF BREAKOUT SESSIONS

## PSYCHOSOCIAL ISSUES

Psychosocial issues affect every aspect of the life of people with SCD. The psychosocial challenges of SCD include:

- For children, missing school and having difficulties making friends.
- For college students, feeling misunderstood and experiencing other social difficulties.
- For adults, being afraid of not living to see their children grow up, facing the continuous expectation of being sick, and experiencing sleep problems.
- Being subjected to stereotypes that affect SCD treatment.
- Due to stigma and lack of provider education, being issued different treatments for pain than those used for pain in patients with cancer.
- Enduring a lack of psychological assessments and referrals by primary care providers.
- Needing access to integrated treatment programs that include behavior health support.
- Facing denials by the Social Security Administration of requests for disability assistance.

Breakout group participants identified the following research questions:

- What are the underlying causes of depression in patients with SCD?
- What causes insomnia in patients with SCD and what treatments are effective?
- What are the psychosocial and physical effects of bone marrow transplants?
- How can the knowledge of SCD among practitioners and the general public be increased?
- How can the segmentation of the health care system be reduced to increase the use of interdisciplinary, holistic approaches in SCD treatment?
- What is the impact of Medicare reimbursement on SCD care?
- How can we prevent cognitive defects that interfere with receipt of psychosocial support?
- Is the main concern during the transition to adulthood the transition of care or the increased risk of death?

## SCD 101

The first SCD 101 session began with a presentation by Wanda Whitten-Shurney, M.D., from the Children's Hospital of Michigan. The second session included a presentation by Harvey Luksenburg, M.D., from NHLBI. Major points made were as follows:

- Prenatal diagnosis of SCD through amniocentesis or chorionic villus sampling is important in planning and becoming conversant about all available options.
- The complications of SCD include pain, infection, acute chest syndrome, stroke, fatigue, ulcers, jaundice, and in children, bedwetting and delayed puberty and growth.
- The risks of blood transfusion include iron overload, infections transmitted through donated blood, and hemolysis (a breaking down of transfused blood cells by the body's immune system).
- Infants and young children with SCD should be treated with penicillin twice a day until age 5.
- Hydroxyurea is the only FDA-approved treatment for SCD to date, but its long-term effects are not known.

## GLOBAL IMPACT OF SCD RESEARCH

Norma Lerner, M.D., M.P.H., and Swee Lay Thein, M.D., D.Sc., both of NHLBI, gave an introductory presentation. They explained that about 14.2 million babies with SCD will be born worldwide between 2010 and 2050. More than 75 percent of SCD births occur in Africa, and about 50 to 80 percent of children with SCD die before age 5 in Sub-Saharan Africa. The United States has a strong research infrastructure but a much lower rate of SCD. The United States and African nations therefore need to work together.

Points made during the breakout group discussion included the following:

- Spreading newborn screening to more countries is feasible.
- Programs in the United States and United Kingdom are not doing a very good job of informing parents that they have sickle cell trait or educating people about how to respond to such information.
- “Point-of-care” testing (e.g., using saliva) could be a major development in SCD.
- Support is needed for SCD researchers and data centers based in Africa.
- Some approaches that are effective in the United States might not work as well in Africa.
- The stigma of SCD and sickle cell trait is a problem in African countries and many other countries.
- Access to transfusions has increased in major cities in Africa, but small towns across the continent still lack access.
- Some programs in developing countries have had good results using hydroxyurea to treat SCD without blood transfusions.
- Survival rates for SCD are much poorer in Sub-Saharan Africa than in the United States or United Kingdom.
- National leadership is needed to bring together people with SCD, family members, SCD organizations, and other SCD stakeholders.

## DIGITAL AND SOCIAL MEDIA BEST PRACTICES

Participants offered the following suggestions for using social media to promote research participation and to create awareness of SCD:

- Follow and repost the messages of researchers, physicians, and organizations.
- Develop a campaign with built-in “spikes” on social media (e.g., by having National Football League players wear red once a year).
- Determine the engagement points that persuade the audience to act and react.
- Develop a common agenda and strategy (e.g., to increase awareness, research dollars, and other support).
- Ask stakeholders to create short Vine videos and send out tweets about SCD gatherings.
- Use social media to spark national debates (sometimes starting with a single tweet).
- Host social media events (e.g., Facebook or Twitter chats) with partners that are not necessarily part of the SCD community.
- Always use the hashtag #sicklecell.

## MAKING CONNECTIONS AND SHARING KNOWLEDGE

The goals of these breakout sessions were to identify important needs of the SCD community, ways to learn from each other and leverage each other’s work, and action items.

Challenges that the SCD community faces are:

- The community does not speak with a single voice.
- Obtaining funding for SCD is difficult.
- The SCD community is unaware of all of the SCD research that is taking place. Raising awareness would increase enrollment in clinical trials.
- Many patients with SCD do not receive adequate information about hydroxyurea from the research community.

SCD Forum action steps are as follows:

- Follow the models of other chronic diseases, such as cancer and heart disease, by developing strong national organizations supported by all stakeholders.
- Develop common agendas for treatment, advocacy, research, pain, fatigue, and psychosocial issues for the entire SCD community.
- Work with the entire SCD community to distribute the message as far and wide as possible (possibly using social media), beyond NIH (e.g., into the academic research community), to help shape the research agenda.
- Translate research results into plain language.
- Sponsor more forums like this one for SCD community members to learn and gather information to take back to their own communities.
- Create partnerships between the research community and persons with SCD.
- Create more strategic approaches for asking legislators and organizations for assistance.

# APPENDIX 2: SOCIAL AND TRADITIONAL MEDIA OUTREACH RESULTS

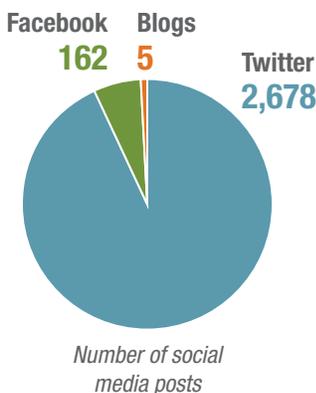
## Overview

On behalf of the National Heart, Lung, and Blood Institute (NHLBI), PR Strategists and ICF conducted traditional and social media outreach to promote the Institute’s forum, “Engaging the Community: Developing Solutions,” held June 25–26, 2015; promote World Sickle Cell Awareness Day on June 19; and raise awareness about sickle cell disease (SCD).

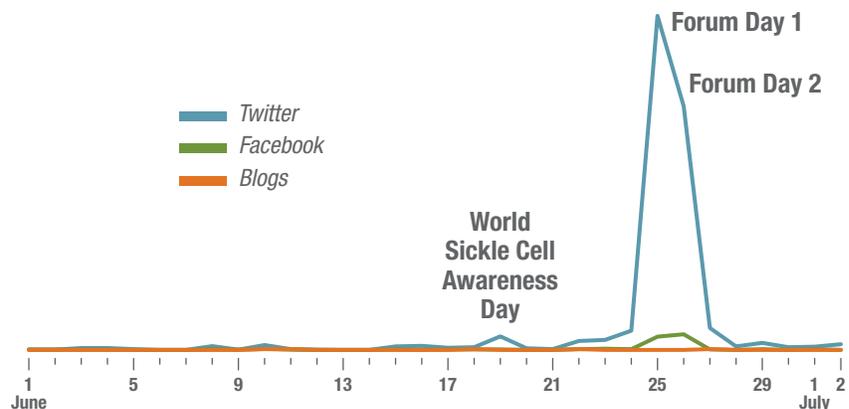
## Social Media

### At-a-Glance: Forum Social Media Activity (June 1–July 3)

#### PLATFORM ACTIVITY



#### SOCIAL MEDIA ACTIVITY BY DATE



### TOP 5 SOCIAL MEDIA INFLUENCERS



**Hertz Nazaire (@NazHz):** An artist and sickle cell disease awareness advocate with **1.1M** followers, Nazaire posted **37** tweets about the Forum.



**P Mimi Poinsett MD (@yayayarndiva):** A pediatrician with **10.6K** followers, Dr. Poinsett posted **242** tweets about the Forum.



**Cleverly Changing (@Cleverlychangin):** A blogger and sickle cell advocate with **8.9K** followers, Cleverly Changing posted **21** tweets about the Forum.



**Shakir Cannon (@BLOODBORN):** A sickle cell activist with **8.7K** followers, Cannon posted **23** tweets about the Forum.



**Judy Stone (@DrJudyStone):** An internal medicine and infectious disease doctor and contributor to Forbes with **4.8K** followers, Dr. Stone posted **67** tweets about the Forum.

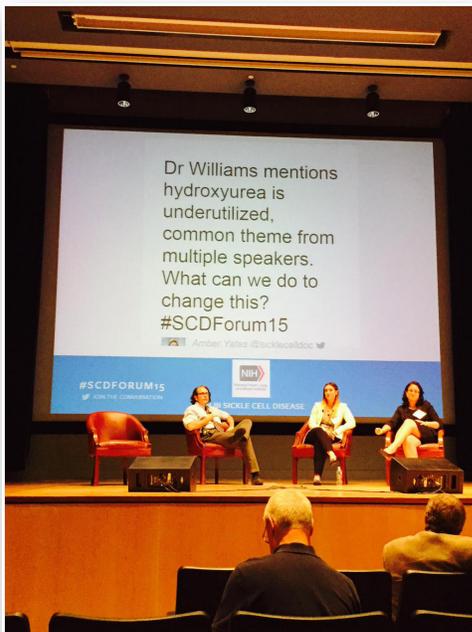
## TOP 5 SOCIAL MEDIA POSTS



## SOCIAL MEDIA AT THE EVENT

- Twenty-six tweets were featured during the event (including comments and questions).

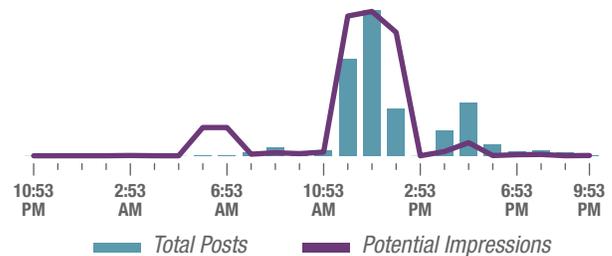
### TweetWall



## LIVE-TWEETING DURING THE FORUM

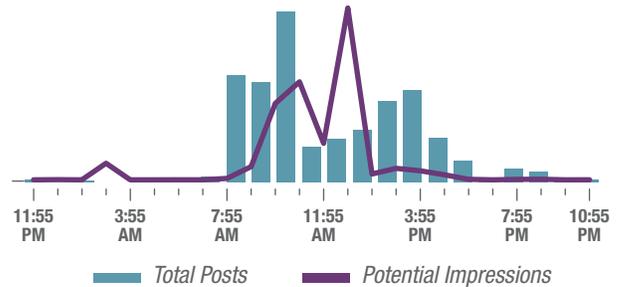
### June 25, Forum Day 1

- Total tweets: **599**
- Total potential impressions: **19,673,209**



### June 26, Forum Day 2

- Total tweets: **663**
- Total potential impressions: **30,267,780**



## TOP INFLUENCERS AND MOST ACTIVE PARTICIPANTS DURING THE FORUM

### Most Followed Users

Number of followers

Hertz Nazaire	1,122,826
Francis S. Collins	47,716
NIH NHLBI	46,621
Howard University	37,089
American Society of Hematology (ASH)	11,768

### Most Active Users

Updates/person

P Mimi Poinsett MD	188
NIH NHLBI	129
Jennifer Thorp	92
Amber Yates	75
Judy Stone	60

## #SCDFORUM15 HASHTAG PERFORMANCE (JUNE 22–JUNE 26)

#SCDForum15  
**1,639**

#sicklecell  
**855**

#sicklecelldisease  
**65**

#sicklecellchat  
**17**

#OBSSRat20  
**188**

TOTAL MENTIONS

## NHLBI SICKLE CELL DISEASE FORUM TWITTER ACTIVITY

- **251** clicks on tweet links
- **862** retweets
- **31** replies
- **781** favorites
- **212,502** potential impressions

*Top engaged tweet:*



**Summary of Twitter Advertising (June 19–24)**

- **91,243** impressions
- **37** clicks on the link
- **14** retweets
- Total: **\$299.05**



## NHLBI SICKLE CELL DISEASE FORUM FACEBOOK ACTIVITY

- **3,250** clicks on post links
- **1,123** likes
- **116** shares
- **35** comments

*Top engaged post:*



**Summary of Facebook Advertising (June 17–22)**

- **12,089** impressions
- **3** clicks on the link
- **31** shares
- **3** comments
- **270** likes
- Total: **\$190.28**



## Traditional Media

A matte article addressing **5** key facts about SCD was pitched directly to African American weeklies in Washington, DC, and five key markets, including those in New York and Atlanta, which garnered **459,114** media impressions.

## MATTE ARTICLE



- The Informer
- Harlem World Magazine
- Weekly Challenger
- Heart & Soul
- Houston Style Magazine

## RADIO HIGHLIGHTS

- An Audio News Release with key facts about SCD aired for **2** weeks in June and on World Sickle Cell Awareness Day during nationally syndicated “The Yolanda Adams Morning Show” and “The Nightly Spirit with Willie Moore Jr.,” garnering **1,184,000** impressions.
- An interview with NHLBI Director Dr. Gary Gibbons on “The Yolanda Adams Morning Show” aired on World Sickle Cell Awareness Day, reaching **1.4 million** listeners.

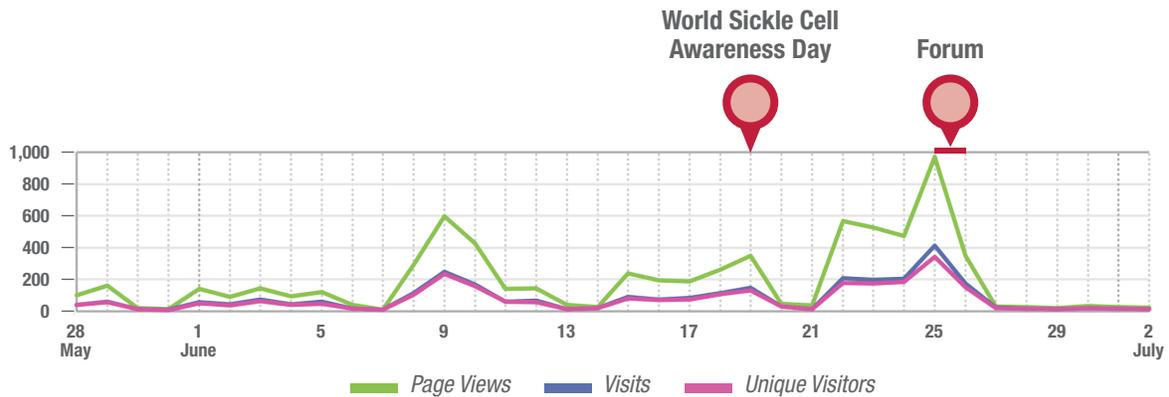
**RADIO**  
**ONE**  
THE URBAN MEDIA SPECIALIST



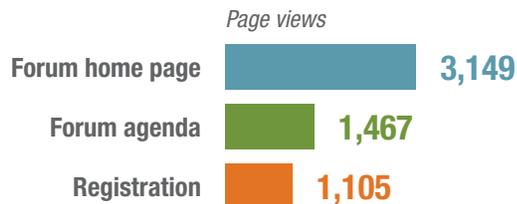
## MEDIA ADVISORY METRICS/HIGHLIGHTS

- A media advisory to promote the forum was disseminated through PR Newswire and picked up on **244** news websites, with a potential audience of **22,123,000** unique visitors. The advisory was featured on AP Newsroom.com, Black Enterprise, Market Watch, Reuters, and Bloomberg News, and more than **120** broadcast news websites.
- The media advisory also received social media attention with **10** tweets, **2** Facebook shares, **1** LinkedIn post, and **1** Google+ share.

## NHLBI Website Traffic



### MOST POPULAR WEB PAGES RELATED TO THE FORUM



### TOP REFERRAL SOURCES

