2019 Access-to-Care Summit

The Journey to Optimal SCD Access to Care

Jonathan Sorof MD
SVP, Medical and Regulatory Affairs
Global Blood Therapeutics
Attendee Feedback from 2018 Access-To-Care Summit

How valuable did you find the content and discussions of SCD access to care models?

- Extremely Valuable: 84%
- Very Valuable: 13%
- Valuable: 3%

Compared to other similar activities that I have participated in, I would rate this program as:

- Better Than I Expected: 90%
- What I Expected: 10%
2018 Suggestions for Future Access-To-Care Meetings

- Public Health/Health system transformation - that's a skillset that needs to be represented in future products/summits
- Concrete plans to include the patient voice
- Discuss Medical Coverage
- Bring in more western US representatives
- More providers to discuss current treatment practice by the adult SCD community
- Medicaid directors - managed medicaid needs to be at the table
- Not enough said about non-hematologist providing primary care/specialty care
2019 ATCS Attendee Profiles
N=98

- Healthcare Professional: 58
- Patient Advocacy: 34
- Other (e.g., Prof Society): 24
- Government Agency: 8
- Payer / Policy: 4
- Pharma (non-GBT)*: 2

* ~ 40 GBT participants/observers
2019 ATCS: Journey to Optimal SCD Access to Care

OBJECTIVES:

☐ Gain a deeper understanding of the evolving stakeholder map related to SCD care access for providers, insurers, advocates and policy makers

☐ Discuss ideas and potential solutions to help the SCD community adapt to the evolving access-to-care landscape
<table>
<thead>
<tr>
<th>Time</th>
<th>Event Description</th>
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<tbody>
<tr>
<td>SEPTEMBER 11</td>
<td></td>
</tr>
<tr>
<td>6:30AM - 7:45AM</td>
<td><strong>BREAKFAST</strong> - 1400 North Restaurant @ The Westin Hotel</td>
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<tr>
<td>8:00AM - 8:10AM</td>
<td><strong>WELCOME</strong></td>
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<tr>
<td>8:10AM - 8:30AM</td>
<td><strong>KEYNOTE ADDRESS</strong>&lt;br&gt;Admiral Brett Giroir, Assistant Secretary for Health at the US Department of Health and Human Services</td>
</tr>
<tr>
<td>8:30AM - 8:50AM</td>
<td><strong>2018 ATCS ROAD MAP DELIVERABLES</strong>&lt;br&gt;Ken Bridges, MD</td>
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<tr>
<td>8:50AM - 9:30AM</td>
<td><strong>GBT’s ACCESS TO EXCELLENT CARE FOR SCD PATIENTS (ACCEL) AWARDEES</strong>&lt;br&gt;&lt;br&gt;Center for Inherited Blood Disorders and Sickle Cell Disease Foundation&lt;br&gt;**James R. Clark Sickle Cell Foundation&lt;br&gt;**Johns Hopkins&lt;br&gt;MAVEN Project (Medical Alumni Volunteer Expert Network)&lt;br&gt;Sickle Cell Foundation of Georgia</td>
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<tr>
<td>9:30AM - 10:00AM</td>
<td><strong>BREAK</strong></td>
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Plenary

**PLENARY SESSION**

10:00AM - 11:30AM

*Addressing the Deficit in Provider Care*
- Wally Smith, MD
- Tyson Pillow, MD
- Wanda Whitten-Shurney, MD

*Navigating the Emerging Complexity of Treatment Coverage*
- John Stancil
- Edem Kojo Ablordeppey, Pharm.D.

*Power of Effective Advocacy: Advancing Policy*
- Heidi L. Wagner
- Senator Tim Scott of South Carolina (video message)
- Conor Sheehy

11:30AM - 12:30PM

**LUNCH**
<table>
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<th>Time</th>
<th>Event Description</th>
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<tr>
<td>12:30PM - 2:00PM</td>
<td>BREAKOUT SESSION 1</td>
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<tr>
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<td>- Addressing the Deficit in Provider Care</td>
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<td>- Navigating the Emerging Complexity of Treatment Coverage</td>
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<td>- Power of Effective Advocacy: Advancing Policy</td>
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<tr>
<td>2:00PM - 2:15PM</td>
<td>BREAK</td>
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<tr>
<td>2:15PM - 3:45PM</td>
<td>BREAKOUT SESSION 2</td>
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<td>- Addressing the Deficit in Provider Care</td>
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<td>- Navigating the Emerging Complexity of Treatment Coverage</td>
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<td>- Power of Effective Advocacy: Advancing Policy</td>
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<tr>
<td>3:45PM - 4:00PM</td>
<td>BREAK</td>
</tr>
<tr>
<td>4:00PM - 4:45PM</td>
<td>2019 ATCS SUMMARY</td>
</tr>
<tr>
<td>4:45PM - 5:00PM</td>
<td>CLOSING REMARKS</td>
</tr>
</tbody>
</table>
Access-to-Care Summit:

2018 Overview and Summary

September 11, 2019
“Of all the forms of inequality, injustice in health care is the most shocking and inhumane”

- Dr. Martin Luther King, Jr.
How Can We Address this Fundamental Issue of Societal Justice?

• The 2018 Access-to-Care Summit
  • Convened by GBT
  • Content developed by a Steering Committee of experts

• Key Principle: Explore solutions, not dwell on problems

• Develop a document that summarizes the deliberations and provides guidance
The document captures the essence of the Summit. Absent, however, is the spirit of the Summit.
Steering Committee
2018 Access-to-Care Summit

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

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

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
Core Elements of Care Access

2018 Summit Focus

Access-to-Care

2019 Summit Focus

People

Programs

Policy

Payers
Programs Essential to Care and Access

- Day Hospital or Clinic
- Pediatric to Adult Transition
- Shared Infrastructure
- Community Outreach
Key Elements to Program Build

- SCD champion/advocate
- Business plan
- Support: patients/advocates, hospital staff and administration, policy leaders
- Data capture
- Periodic assessment
Comprehensive SCD Care: Connecting the Pieces

Adult Day Hospital/Clinic

Shared Infrastructure

Medical Center
General Care Facilities

Pediatric to Adult Transition

Pediatric Care
Expanding the Reach of SCD Expert Care: Brave New World*

*How beauteous mankind is! O brave new world, That has such people in ’t!
- The Tempest
Every man takes the limits of his own field of vision for the limits of the world

Arthur Schopenhauer
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<th>ACCEL Project</th>
<th>Organization</th>
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<td>Strengthening Access to Sickle Cell Care in Southern California (Project SAS-SC)</td>
<td>Center for Inherited Blood Disorders and Sickle Cell Disease Foundation</td>
<td>Mary Brown</td>
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<tr>
<td>Project LEAP Expansion</td>
<td>The James R. Clark Memorial Sickle Cell Foundation</td>
<td>Melodie Hunnicutt</td>
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<tr>
<td>Optimizing Sickle Cell Disease Training Through an Advanced Practice Provider Residency</td>
<td>Sickle Cell Center for Adults at Johns Hopkins</td>
<td>Sophie Lanzkron</td>
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<tr>
<td>Improving Management of Sickle-Cell Disease in the Primary Care Setting</td>
<td>The MAVEN Project</td>
<td>Samrina Marshall</td>
</tr>
<tr>
<td>Sickle Cell Disease for Non-Specialty Health Care Providers</td>
<td>Sickle Cell Foundation of Georgia, Inc.</td>
<td>Milford Greene</td>
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Access-to-Care Summit:

Strengthening Access to Sickle Cell Care in Southern California (Project SAS-SC)

Mary Brown, Sickle Cell Disease Foundation
Diane Nugent, MD, Center for Inherited Blood Disorders
Partnership between The Center for Comprehensive Care and Diagnosis of Inherited Blood Disorders (CIBD) & Sickle Cell Disease Foundation (SCDF)

**CIBD – Center for Inherited Blood Disorders**

**Mission:** To deliver the highest quality comprehensive healthcare to our patients.

**Purpose of the ACCEL grant**
Improve access to knowledgeable clinical care and care coordination services to individuals with sickle cell disease in Southern California through capacity building, education, and telementoring.

**Sickle Cell Disease Foundation**

**Mission:** To improve the whole lives of those living with sickle cell disease across the nation by destroying barriers, cultivating unprecedented partnerships and employing innovative strategies to deliver impactful advocacy initiatives and life-enhancing programs, while fostering new research and therapies to eradicate sickle cell disease.
Project Objectives

Increase understanding of the challenges and concerns that healthcare professionals face (with a special focus on Adult Hematologists, Primary Care Physicians, and Nurse Practitioners) in Southern California when caring for adults and transitioning pediatric patients with Sickle Cell Disease.

Increase the knowledge of healthcare professionals regarding SCD based on priority education needs.

Increase access to information on SCD for healthcare professionals, community based organizations, and government partners throughout California and the Pacific Region. The Pacific Region includes these states: Alaska, Arizona, Hawaii, Utah, Idaho, Nevada, Colorado, Oregon, Montana, Wyoming, New Mexico, California, and Washington.
Outcomes

• An increase in self-perceived knowledge by at least 20% from baseline.
  • A 3-month follow up will be conducted to assess knowledge retention.
  • Between the post-test and 3-month follow up, we anticipate different levels of retention, based upon the level of engagement and also based on participant characteristics (e.g. provider type, level of experience caring for people with SCD).
Contact Information

Center for Inherited Blood Disorders
Diane Nugent, MD
dianenugent7@gmail.com

Shalini Vora, MPH
svora@c3dibd.org
(714) 221-1222

Sickle Cell Disease Foundation
Mary Brown
maryb@scdfc.org
(909) 743-5226

Jennifer Fields, MPH
jenniferf@scdf.org
(252) 481-3030

Deborah Green
deborahg@scdfc.org
(909) 743-5226
Access-to-Care Summit:

Project LEAP Expansion

Dr. Melodie A. Hunnicutt, CFRE
Today’s Presentation

Institution/Organization Background

• The James R. Clark Memorial Sickle Cell Foundation
  • Located in Columbia, South Carolina, 1972

Access to Excellent Care for Sickle Cell Patients (ACCEL) Grant Overview

• Project LEAP (Locate, Educate, Assess, Place), an initiative to identify unserved and underserved SCD patients in South Carolina initially funded through a HRSA Newborn Screening Follow-Up Grant.

Mission Statement

• To optimize the social, psychological, and physiological well-being of individuals with sickle cell disease and to decrease the incidence of sickle cell disease through genetic screening, counseling, and education
Project Objectives

• **Identify sickle cell patients who are not receiving care** in a medical home and connect them with primary and specialist providers, with special emphasis on patients living in rural areas;

• **Provide follow-up care coordination services** to this identified patient population, including assessment and intake and patient education services;

• **Target emerging adults** transitioning from pediatric to adult care and increase the number of adolescents and young adults seen by an adult SCD care provider; and

• **Increase the number of statewide partnerships** (formal and informal) with hospitals, healthcare systems, primary care providers, and other community-based medical and human service organizations to assist with patient identification and recruitment
Outcomes

Short-range Outcomes

• Identify and serve a total of 80 patients over the funding term of one year
• Provide patient assessment, referral, placement, and education services to 80 total patients
• Assist 8 emerging adults to transition from pediatric to adult care services
• Increase by 12 the number of community partnerships statewide

Medium-range Outcomes

• Patients have greater continuity of care through a new network of primary and specialty medical providers
• Patients are empowered to better manage their own healthcare and disease process
• Patients have access to new and innovative community and social resources and supports

Long-range Outcomes

• Patients are able to live longer and healthier lives and have a higher quality of life on a daily basis
Contact Information

Dr. Melodie Hunnicutt, Executive Director

James R. Clark Memorial Sickle Cell Foundation
1420 Gregg Street
Columbia, SC 29201

E-mail: sicklecell@sc.rr.com

Office phone: (803) 765-9916
Cell phone: (803) 447-8686
Access-to-Care Summit:

Optimizing Sickle Cell Disease Training Through an Advanced Practice Provider Residency

Sophie Lanzkron, MD, MHS
Sickle Cell Center for Adults at Johns Hopkins

Provides comprehensive care, including:
- Regularly scheduled outpatient visits
  - Disease modifying therapy
  - Pain management
  - High utilizer clinic
  - Patient education
  - Community health workers
  - Nurse case management
  - Social work support

Sickle Cell Infusion Center (SCIC)
- Established in 2008
- Alternative to the ED for the management of acute painful events
- Decreased admissions and readmissions at Hopkins and throughout Baltimore

Highly skilled APPs
- Manage all acute visits in the SCIC
- See every adult admitted anywhere at JHH with SCD daily

Close ties with the sickle community
- Longstanding relationships with a number of CBOs
- Support groups

MLR Document Number: M-UNB-US-00047 V1.0
Access to Care

- Nation-wide shortage of benign hematologists who care for adults with SCD
  - Lack of reimbursement
  - High level of coordination of care needed for the SCD population
  - Not emphasized in many hem/onc training programs
- Greatly limits access to high quality care resulting in poor outcomes
- Need to consider new models to expand access to care out of tertiary care and into communities where patients live
  - Need to develop comprehensive centers that provide team based care led by a knowledgeable provider
Project Goal

- To develop a curriculum to build a strong, well trained advanced practice provider (APP) workforce to care for adults with SCD
  - Establish a curriculum for a residency program for APPs
  - Utilize an assessment tool to validate the APP residency curriculum
  - Create a network of APPs who are engaged in treating those living with SCD
Focus group sessions will provide a detailed understanding of the gaps in knowledge and training that will need to be addressed in an APP residency program. At completion of funding, we will have a detailed APP residency curriculum and assessment tool.

Network of APPs who work in SCD

- Use Project ECHO
- Supportive for new APP’s in the field
- Help establish best practice for the workforce
Contact Information

Sophie Lanzkron
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Mandy David
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Access-to-Care Summit:

Improving Management of Sickle-Cell Disease in the Primary Care Setting

Samrina Marshall, MD, MPH
Chief Medical Officer
The MAVEN Project
The MAVEN Project

Mission

• The MAVEN Project connects volunteer physicians to primary care providers (PCPs) working in underserved clinics through telehealth technology
Project Activities and Goals

Add one new clinic site to The MAVEN Project’s network that is known to serve SCD patients or populations with known sickle cell prevalence.

Inform sickle cell populations in this clinic’s catchment area that they are welcoming SCD patients.

Increase the capacity of The MAVEN Project’s volunteer physician corps in the area of SCD management.

Alert the MAVEN Project clinic sites nationwide that SCD expertise is available.
Project Activities and Goals (continued.)

**Project Participants**
(Institution/CBO/Organization)

- Based on regional SCD prevalence data, we will add one new clinic to our partner network and inform the community of the available SCD expertise
- Add more physician volunteers with expertise in pediatric and adult SCD management
- Inform our national clinic network on the availability of this expertise

**New site**
receives our full scope of services

- Advisory consults with specialists including hematologist specializing in SCD
- Educational Sessions focused on SCD related topics
- Mentoring: supporting other aspects of care such as clinical case review and communicating with challenging patients
- Senior Partnership Manager to optimize the clinic and The MAVEN Project partnership

MLR Document Number: M-UNB-US-00047 V1.0
Project Outcomes

• Improve access to specialty consultations in new MAVEN Project clinic site
• Increase SCD clinical competency by supporting the local PCP in MAVEN Project clinic sites nationwide
• Improve overall job satisfaction for on-site PCPs who see sickle cell patients
• Better manage SCD in the primary care setting
  • Improve health and wellness for sickle cell patients
  • Reduce the need for emergency care
  • Reduce reliance on emergency departments for non-urgent care
  • Reduce unnecessary in-person specialist visits
Resources

The MAVEN Project:
Dr. Samrina Marshall

E-mail: smarshall@mavenproject.org

Phone: (617) 641-9743 x701
Access-to-Care Summit:

Sickle Cell Disease for Non-Specialty Health Care Providers

Milford W. Greene, Ph.D., M.P.H.
Sickle Cell Foundation of Georgia, Inc.

• Atlanta, Georgia

Mission

• The mission of the Sickle Cell Foundation of Georgia is to engage, educate, and energize the community to improve the quality of life for people affected by sickle cell disease.
Project Objectives/Goals

Conduct four (4) sickle cell disease training seminars for non-specialty health care providers to build capacity to treat sickle cell disease.

Conduct at least one of the provider seminars at a Federally Qualified Health Center.

Disseminate 250 provider toolkits to primary health care and emergency department providers to improve access to information on informed appropriate care.

Community Health Workers (CHWs) will assist fifty (50) young and older adults to generate a travel letter with their hematologists as part of a toolkit to enhance patient-provider communication.

Disseminate 75 toolkits to young and older adults with information to strengthen relationships between patients and providers.
Project Outcomes

- **200** non-specialty medical providers will receive training on appropriate evidence based treatment of sickle cell disease.

- **75%** of participating medical providers will demonstrate an increase of knowledge about sickle cell disease and the treatment of sickle cell disease after training.

- **65%** of participating medical providers will report feeling more comfortable treating individuals with sickle cell disease after training.

- **75%** of individuals receiving patient tool kits will report using the travel letter or information in the toolkit at least once after receipt.

- **50%** At least 50% of individuals using information or letter from the tool kit will report easier communications with providers.
Contact Information

Milford Greene, PhD, MPH
Director of Clinical Services

Sickle Cell Foundation of Georgia, Inc.
2391 Benjamin E. Mays Drive, SW
Atlanta, GA 30311

mgreene@sicklecellga.org
Cell: 610-513-4987
Access-to-Care Summit:

Deficit in SCD Provider Care: Defining the Problem
Access-to-Care Summit:

Medical Care for Adults With Sickle Cell Disease: Improving From Not Very Much & Not Very Good

Wally R. Smith, MD
Florence Neal Cooper Smith Professor of Sickle Cell Disease
Vice Chair for Research, Division of General Internal Medicine
Virginia Commonwealth University
Failed Adult SCD Care

Mortality Rate per 100,000 African American population

- Adult rate (> 19 years of age)
- Child rate (≤ 19 years of age)

ASH SCD Report Card, 2015

Access to Care*
- 3.7

Training, Professional Education**
- 3.2

*Health insurance, availability of knowledgeable healthcare providers, provider experience, geography, economic status, coexisting conditions.

**Not enough healthcare providers with SCD expertise, unpredictable and persistent pain, complications → management challenges.

Primary care feels they have inadequate SCD background.
Adult SCD Care: Not Very Much, Not Very Good

- All ASH Members: Few (~1,100/27,000) with SCD exposure

- FL, NC oncologists seeing SCD patients
  - Overall: 57% seen in hospital; 43% seen overall
  - Management per NIH guidelines: 35%

- PCPs with SCD patients in NC
  - Comfortable with their SCD patient number: 73%
  - Frequent contact with SCD specialist: 33%
  - Aware of NIH guidelines: 34%

Ecology of SCD Care

77,000 pts with SCD (census, Hardy Weinberg equation est.)

- PCP workforce = 306,100 (fed est.)
- PCPs have 0-1 SCD patients/MD (est., anecdotal)

CSCCs, specialists care for 10,000 patients (est., NIH)

28% of patients managed by specialists are on HU (PiSCES data)
Part of the Solution: Improved Pediatric-to-Adult Care Transition

Providers who care for youth/young adults throughout the lifespan complete both sets of core elements without transfer process.
Part of the Solution: Adult Patient-Centered Medical Home

- Safety & Quality
- Coordinated & Integrated Care
- Whole Person Orientation
- Personal Physician
- Physician Directed Practice
- Enhanced Access
SCD Medical Home and Medical Neighborhood: A Complex Ecosystem

**SCD Medical Home**

- **Home**
  - Physical Therapy
  - Home Healthcare Nurse
  - Family/Caregiver

- **Community**
  - Support Groups
  - Patient Navigators
  - Pharmacy
  - Community Partners (SDOH)
    - CBOs
    - Housing, food, etc.

- **Other Outpatient Care**
  - Specialty Clinics
    - Substance Abuse (MOTIVATE)
    - PEDS Transition Programs
    - Psychiatry Neuropsychiatry
    - BMT/Transplant
  - Procedure Center
    - Pheresis
    - Infusion
    - Transfusion Medicine

- **Acute Care (Tertiary Hospital)**
  - Acute Care (Community Hospital)
  - Transfer Center
  - Emergency Dept
    - CDU
  - Inpatient
    - Hospital Medicine
    - Graduate Med Edu Program
    - Acute Pain Consult Team
    - Substance Abuse Consult Team
    - Hematology and Oncology Consults

- **Post-Acute Care**

*Out of Scope
- Other Medicine Specialties

BMT, bone marrow transplant; CDU, clinical decision unit; SDOH, Social Determinants of Health.

MLR Document Number: M-UNB-US-00047 V1.0
ASH SCD Implementing Comprehensive Care Workshop: Needs Survey

• Prior ASH All-Member Survey: approximately 1,100 with SCD exposure/27,000 members

• Comprehensive care survey
  • August 2018
  • Viewed by 547 members
  • 50% completion rate (80 completed/160 started)
  • All completers in US
Innovative Program Prevalence

Within the health system in which you primarily work, what is your experience working with the new SCD models?

- Infusion Center: 56.75%
- Day Hospital: 49.00%
- Outpatient Clinic: 66.25%
- SCD Inpatient Unit: 50.00%
- SCD Medical Home: 49.25%
- Other: 40.25%
Interest in SCD Workshop on Centers to Improve Adult Care

• Would you be interested in attending the workshop described above? N = 80

- Yes: 87.50% (70)
- No: 6.25% (5)
- I don't know: 6.25% (5)
Ability to Implement an Adult Medical Home

• How confident are you in the following areas? N = 80

- Evolving care from episodic to longitudinal care in outpatient setting: 73.25%
- Leadership and change management: 56.00%
- Patient community engagement: 54.75%
- Advocacy and alliance development: 51.25%
- Business planning and financial analysis: 35.75%
 Desire for Help From Colleagues in SCD Care

- Welcome extramural colleague collaboration to advocate for a new SCD model in your institution
- Ongoing coaching to implement a new SCD model in your health system

**Yes** 81.25%

**I don’t know** 15%

**No** 3.75%

**Very Important** 35%

**Important** 23.75%

**Moderately Important** 23.75%

**Slightly Important** 15%

**Not Important** 2.5%
Emergent Care for Adults With Sickle Cell Disease:
Fighting the Bias and Stigma

M. Tyson Pillow, MD, MEd
Association Professor
Vice Chair for Education, Department of Emergency Medicine
Baylor College of Medicine
The New England Journal of Medicine

**Special Article**

THE EFFECT OF RACE AND SEX ON PHYSICIANS’ RECOMMENDATIONS FOR CARDIAC CATHETERIZATION

Top Tips to Help Optimize Care in the Emergency Department

• Understand the biases and stigma that may be present
• Have a clear, complete conversation with your provider
• Be honest and open about your care needs
• Connect your ED providers with providers who know you
• Educate yourself and be the expert in your own care
Access-to-Care Summit:
Engaging Federally Qualified Health Centers

Wanda Whitten-Shurney, MD
CEO/Medical Director
Sickle Cell Disease Association of America,
Michigan Chapter
U.S Department of Health & Human Services

HRSA
Health Resources and Services Administration

NICHQ
National Institute for Children’s Health Quality
Treatment Demonstration Grant
   Increase # of physicians caring for patients with SCD
   Increase # of physicians prescribing hydroxyurea

SCDAA
Newborn Screening Grant
   Locate patients – community health workers
   Get connected – close to 5,000 enrolled
Federally Qualified Health Center (FQHC)

• Comprehensive primary care and preventive care
• Health and oral care
• Mental health/substance abuse services

Mission

• To improve the health status of underserved populations in their targeted communities without regard to ability to pay or health insurance status
Key Healthcare Challenges for People With SCD

- Awareness
- Young people don’t know their trait status
- Only 2 new medications in 20 years
- No universal cure
- Pediatric vs adult care
  - Lack of hematologists and PCPs
  - Stigmatization
  - “Opioid crisis”
Clinical Trials

Cell adhesion
• Rivapensil – GMI 1070 (RESET) -- interfere with adhesion to selectin
• Sel G2 – interfere with adhesion to selectin
• Poloxamer 188 (EPIC) – stabilizes damaged membranes making less sticky

Inflammatory pathways
• NKTT120 – iNKT cell inhibitor
• Regadenoson – iNKT inhibitor

Upregulation of hemoglobin F
• HQK-1001 – induce fetal hemoglobin
• panbinostat

Hemoglobin polymerization and sickling
• AES-103 – binds hemoglobin to prevent polymerization
• Voxelator (GBT440) – increased oxygen affinity

Nutrition
• Omega 3 Fatty Acid (SCOT Trial)

Platelet activation
• DOVE – (Prasugrel) – interferes with platelet response
• Hestia - Ticagrelor –Phase II
Type of sickle cell  ____ SS  ____ SC  ____ Sbeta thal zero  ____ Sbeta thal plus

Other ______________________

Usual hemoglobin level ________________

Blood type ___________________________

Allergies:

• Medications
• Hospitalizations
• Transfusion history
• Surgical history
• Diagnostic studies
• Medical appointments

Passport – to Health and Wellness
NHLBI Guidelines:
Evidence-Based Management of Sickle Cell Disease, 2014.

ASH Resources:
• Management of Acute Complications of Sickle Cell Disease
  A Pocket Guide for the Clinician

• Health Maintenance and Management of Chronic Complications of Sickle Cell Disease
  A Pocket Guide for the Clinician

• Hydroxyurea and Transfusion Therapy for the Treatment of Sickle Cell Disease
  A Pocket Guide for the Clinician

STORM TeleECHO:
http://sickleecho.org/
Matching Community Health Workers With Specific FQHCs Improves Outreach and Education Possibilities

**Number of SCD Patients**
- □ 0-30 patients
- ■ 31-200 patients
- □ 201-600 patients
- □ > 600 patients

**Cities of Focus for SCDA of Michigan CHW Outreach**

**FQHC Distribution Across Michigan**

- 52 FQHCs that are in the vicinity of 70% of the SCD patients

**CHW, community health worker location**
Lessons Learned

• Review, prioritization and targeting of FQHC list should align with patient population concentration

• Focus strategically on areas of the state where there is a high population of at-risk individuals (eg, Hispanic patients)

• Efforts to schedule presentations are labor intense

• The information is well received once you get your foot in the door

• Follow-up is key to ensuring patient access

• Engagement of other HCPs is important (eg, nurse practitioners and physician assistants)

Process could be beneficial to other states = more patients access primary care
It takes a Village
Access-to-Care Summit:
Navigating the Emerging Complexity of Treatment Coverage
Orphan Disease Landscape

Orphan diseases affect an estimated **25 million** people in the US

50% of orphan diseases affect the pediatric population

Additional R&D support programs and nonprofit research affiliates were created to spur investment

The first novel therapy for cystic fibrosis came to market in 1993

About **30%** of phase 3 studies of orphan drugs fail due to enrollment challenges

Urgency of getting drugs to patients: Subpart H—Accelerated Approval of New Drugs for Serious or Life-Threatening Illnesses

Out of the 36 Subpart H approvals in the last 5 years, 30 were for orphan diseases
The Role Key Stakeholders Play in the Coverage of Orphan Drugs

What is the role of the:

- Prescribing physician
- Manufacturer
- Patient and community-based organizations
Presenters

John Stancil
Pharmacy Director
North Carolina Medicaid

Edem Kojo Ablordeppey, PharmD
Sickle Cell Patient and Rutgers Univ. Pharmaceutical Industry Fellow
Sickle Cell Disease: A Medicaid Total Care Perspective

Social determinants of health

Statewide framework and infrastructure to support innovation

Quality of care

Standardized screening to assist patients with unmet health-related resource needs

Statewide resource platform
A Balancing Act: Potential Effect of New Therapies vs Healthcare Budgets

Increasing cost

Limited budget
P&T Review Process: 2 Workstreams

Clinical Workstream
- FDA approval
- Preliminary clinical research
- Physician input
- P&T content development
- P&T review
- PAG review

Business Workstream
- FDA approval
- Market research
- P&T / PAG designation
- CMS contract
- Coverage policy
The Role of the Manufacturer in Creating Coverage

- Disease state/prevalence
- Clinical drug review: indications/use, dosage, and administration
- Clinical evidence/outcomes
- Cost/value
- Budgetary impact
- Expectation setting
The Role of the HCP in Creating Coverage

- Place therapy based on clinical evidence/outcomes
- National treatment guidelines
- Site of care
- Appropriate use of management strategies
How to Navigate the System to Get Access to Care: Patient Perspective
Maximizing Access for SCD Patients

Patients with SCD are complex, have multiple comorbidities, and require individualized treatment strategies.

- Allow physicians and patients to make SCD management decisions
- Minimize barriers to patient access to SCD treatments and diagnostics
- Minimize physician burden when prescribing SCD treatments

Patients with SCD would benefit from an individualized treatment strategy that includes new, innovative therapies, either alone or in combination.
Successfully Navigating the System

Patients’ role once SCD drugs are approved

**Understand your healthcare benefits**
Choose the appropriate health plan

**Identify resources available for SCD patients**
- Government programs
- Manufacturer programs
- Advocacy support

**Obtain prior authorization and appeal a denial**
Work with your HCP on the appeal and denial process

What can patients do to successfully gain coverage?
Access-to-Care Summit:
Importance of Effective Advocacy

Heidi L. Wagner
SVP, Government Affairs and Policy
Global Blood Therapeutics
Importance of Advocacy

• Necessary and recognized element of our democracy to ensure a truly representative government
• Necessary to ensure appropriate education of policymakers and staff
• Highly regulated as a profession, but do not need to be a professional or registered lobbyist to have a meaningful impact

“Congress shall make no law respecting an establishment of religion, or prohibiting the free exercise thereof; or abridging the freedom of speech, or of the press; or the right of people peaceably to assemble, and to petition the Government for a redress of grievances.” [sic]

First Amendment, U.S. Constitution
5 Key Principles of Effective Advocacy

• Do Your Homework
• Tell the Truth and Get Personal Where Appropriate
• Keep It Simple and Conversational
• Accept Your Friends Where You Find Them
• Be Patient and Flexible, but Persistent
Sickle Cell Disease (SCD) Policy Focus

- Issues impacting SCD patient access to high quality, coordinated health care services and therapies
  - SCD (and other Heritable Blood Disorders) Research, Surveillance, Prevention and Treatment Act
  - Greater focus on availability of health care providers to treat SCD patients
  - Greater federal/state funding for providing coordinated care services
  - Focus on education of ED personnel

- Issues impacting SCD patient access to new medicines in Medicare and Medicaid programs
  - High percentage of SCD patients are public program participants
Breakout Sessions

1. **Principles**
   - Discuss principles and strategies for effective advocacy

2. **Preparation**
   - Discuss how to prep for effective, high impact meetings

3. **Practice**
   - Practice advocacy skills with former Capitol Hill staff
     - Darren Willcox, Principal of W Strategies, former House Republican leadership
     - Eden Shiferaw, Sr. Director, NVG Strategies, former House Democratic staff
Senator Tim Scott (R-SC)

- 2nd Term (appointed in 2012)
- First African American to be a U.S. Senator from South Carolina and the first from the Southern U.S. since Reconstruction
- Senate Leader on Health Policy
  - Committee on Finance
  - Committee on Health, Education, Labor and Pensions (HELP)
- Senate Leader on Sickle Cell Disease Policy and Driver of 2018 Reauthorization of the SCD Research, Surveillance, Treatment and Prevention Act
- Health Policy Advisor, Conor Sheehey