Increasing Shared Decision-Making and Clinical Trial Participation for People Living with Sickle Cell Disease

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The Sickle Cell Disease Association of America, Inc.
At the conclusion of the activity, attendees will be able to:

1. Describe the barriers to clinical trial participation.
2. Identify effective strategies to increase shared decision-making and clinical trial participation.
3. Summarize current clinical trials, including those studying symptom management, transplant and novel therapies.
4. Identify educational programs and resources to support patients and caregivers learn about and join clinical trials.
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Sickle Cell Disease: Shared Decision-Making and Clinical Trial Participation

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Medical Disclosure

This activity will include a discussion of off-label treatment and investigational agents not approved by the FDA for use in the US.

No medical disclosure
Current Clinical Trials for SCD: Curative Therapies

- Dramatic increase in availability of clinical trials for SCD
- Over 127 clinical trials registered in clinicaltrials.gov
- 35 interventional clinical trials with NIH/Industry sponsor
- Recruitment and retention of research participants remains a challenge
Current Clinical Trials for SCD: Curative Therapies

Bone Marrow Transplantation vs Standard of Care in Patients With Severe SCD (BMT CTN 1503). First comparison of BMT vs. Standard of care in SCD

Nonmyeloablative Haploidentical Peripheral Blood Mobilized Hematopoietic Precursor Cell Transplantation for SCD. NIH study of nonmyeloablative BMT in Adults
Current Clinical Trials for SCD: Curative Therapies Using Haploidentical Donors

Haploidentical Bone Marrow Transplantation in Sickle Cell Patients (BMT CTN 1507). First Multicenter study of haploidentical BMT for SCD

Haplo T-Cell Depleted Transplantation in High-Risk Sickle Cell Disease (HaploSCD) haploidentical BMT for SCD with ex-vivo T cell depleted HSCs

Mesenchymal Stromal Cells for Haplo-Hematopoietic Cell Transplantation for SCD haploidentical BMT for SCD with autologous MSCs to improve outcomes
Current Clinical Trials for SCD: Curative Therapies Using Expansion of Umbilical Cord Blood

Allogeneic SCT of NiCord®, UCB-Derived Ex Vivo Expanded Stem and Progenitor Cells, in Patients With Hemoglobinopathies. Expansion of umbilical cord blood cells to increase availability of donors.

Allogeneic SCT of CordIn™, in Patients With Hemoglobinopathies. Expansion of umbilical cord blood cells to increase availability of donors.
Current Clinical Trials for SCD: Gene Therapies

A Phase 1/2 Study Evaluating Gene Therapy by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with the LentiGlobin BB305 Lentiviral Vector in Subjects with Severe SCD

Gene Transfer for Patients With Sickle Cell Disease

Stem Cell Gene Therapy for Sickle Cell Disease
Current Clinical Trials for SCD: Novel Agents

The Role of Endothelin-1 in Sickle Cell Disease

A Study to Evaluate Safety, Pharmacokinetic, and Biological Activity of INCB059872 in Subjects With Sickle Cell Disease

Study to Evaluate the Effect of GBT440 Administered Orally to Patients With Sickle Cell Disease (GBT_HOPE) (GBT_HOPE)

Pharmacokinetics and Pharmacodynamics Study of SEG101 (Crizanlizumab) in Sickle Cell Disease (SCD) Patients With Vaso- Occlusive Crisis (VOC)

A Study of IMR-687 in Adult Patients With Sickle Cell Anaemia (Homozygous HbSS or Sickle-#0 Thalassemia)
Current Clinical Trials for SCD: Novel Agents

Study of Efficacy, Safety and Tolerability of ACZ885 (Canakinumab) in Pediatric and Young Adult Patients With SCD. Anti-IL-1β monoclonal antibody

Safety Of Rivipansel (GMI-1070) In The Treatment Of One or More Vaso-occlusive Crises In Hospitalized Subjects With SCD. Pan-selectin inhibitor Rivipansel

Fetal Hemoglobin Induction Treatment Metformin (FITMet). Metformin stimulate increase FOXO3 expression

A Study of the Effect of IW-1701, a Stimulator of Soluble Guanylate Cyclase (sGC), on Patients With SCD. NO metabolism regulation

Preventing SCD Kidney Disease. Losartan to prevent renal disease
Current Clinical Trials for SCD: Hydroxyurea Management and Supportive Care

Enhancing Use of Hydroxyurea In Sickle Cell Disease Using Patient Navigators (SHiP HU)

Hydroxyurea Adherence for Personal Best in Sickle Cell Disease (HABIT): Efficacy Trial

Hydroxyurea Management in Kids: Intensive Versus Stable Dosage Strategies

Hydroxyurea to Prevent Brain Injury in Sickle Cell Disease (HUPrevent)
Current Clinical Trials for SCD: Supportive Care

Topical Sodium Nitrite in Sickle Cell Disease and Leg Ulcers

Computerized Cognitive Behavioral Therapy Assisted Life Management for Pain in Sickle Cell Disease (CALM-SCD)

Laboratory-based Hypnosis Intervention on Pain Responsivity in Adolescents With Sickle Cell Disease

Sleep and Pain in Sickle Cell Disease
Barriers to Participation in Clinical Trials for SCD

- More than half of adults, and parents of children with SCD, are not interested in participating in studies involving taking medication or hospital admission
- Previous research participation associated with interest in subsequent research participation
- Lower health literacy associated with less interest in some research types
- Poor communication between providers/researchers and patients
- Historic mistrust of research

Cronin et al AJH 2016
How to Enhance Clinical Trial Participation and Shared Decision Making

• Factors informing caregiver decision: Lessons from Uptake of Hydroxyurea (Hu)
• Acceptable level of risk varies among individuals
• Risk perception depends on risk presentation
• How can physicians facilitate shared decision making
Factors Considered by Caregivers in Making Decision: Lessons from Uptake of HU

- Caregivers who chose HU:
  - Perceived SCD in their child to be severe
  - Concerned about prevention of long term complications/mortality

- Caregivers who refused HU:
  - Did not perceive SCD to be severe
  - More concerned about short term well being of child
  - Admitted to less knowledge about SCD

Creary et al BMC Res Notes 2015; 8:372
Factors Considered by Caregivers in Making Decision: Lessons from Uptake of HU

- All had relationship of trust with HCP
- Caregivers who chose HU engaged HCP with questions
- Caregivers who refused HU did not engage HCP with questions
- All sought information on the internet
- Were guided by past experience with HU
- Sought other’s experience with HU
- Willing to share their own positive experience with HU

Creary et al BMC Res Notes 2015;8:372
Factors Considered by Caregivers in Evaluation of Any Treatment Option

Acceptance of Mortality Risk Varies Among Patients and Caregivers


Risk Acceptance May Depend on Method of Risk Presentation

Number of families willing to consider HU with low or high level of risk following three different methods of presentation of risk


1/3 – one third,
2/5 – two fifths,
2 3/5 – two and three fifths,
1/2 – a (one) half,
1/4 – a (one) quarter.
Why Shared Decision Making

- Ethical imperative to share important decision making with patient
- Addresses barriers in informed consent
- Can overcome health disparities
- Improve quality of medical decisions to achieve outcomes most important to patient
Shared Decision Making

- A collaborative process between patient and healthcare provider
- Active participation of the patient
- A two way flow of information between two experts, the patient and the provider
- Accurate description of all options and their pros and cons
- Assess patient’s values and preferences
- Assist patient in making a decision giving adequate time and resources
- Evaluate patient decision
Gaps in Utilization of Disease Modifying Therapy and Shared Decision making in SCD

- Hydroxyurea, chronic blood transfusion and HCT can modify disease and reduce complications, and improve survival.
- Widespread lack of awareness
- Prototypic model for health disparities
- Disease modifying therapies are underutilized
- Avoidable morbidity and premature mortality
What are the Attitudes of SCD Hematologists Towards Disease Modifying Therapy and Decision Making

• 36 physician experts in SCD, BMT for SCD recruited at National SCD meetings
• Semi-structured qualitative interviews
• Prompts focused on philosophy regarding disease modifying therapy, approach to decision making, perception of how patients made decisions, decisional needs of patients and recommendations on design of web based decision aid.
• Physician approach to decision making ranged from a collaborative approach to that of a proponent of a particular treatment
• Physician approach was also influenced by the decision, the patient and the severity of the disease

Physician Approach to Discussion of Treatment Options is Variable: May Depend on Disease Severity, Intensity and Urgency of Treatment

Factors Affecting Shared Decision Making for SCD

Institutional practice regarding chronic transfusion, red cell apheresis

Organizational Factors
- Institutional practice regarding referral for BMT
- Insurance coverage of BMT, apheresis for patients on Medicaid or private insurance

Decision Making Interacting Factors

Patient Characteristics
- Severity of SCD
- Level of risk acceptable
- Perceived burden of treatment
- Availability of donor
- Trust
- Adherence
- Impact of SCD on QoL
- Expectations from treatment

Physician Perception of Patient Characteristics
- Psycho-social patient characteristics
  - Burden of caregiving
  - Patient/Caregiver Motivation and Commitment

Decision Characteristics
- Risks and benefits of treatment
- Reversibility of toxicity
- Treatment response
- Variability in practice
- Urgency of decision

Physician fostering patient involvement in decision making

Patient Knowledge
- Options
- SCD Values

Shared Decision Making

Patient/Caregiver Values
- Physician fostering patient involvement in decision making

Physician relationship with BMT team
- Access to BMT center

Institutional practice: HU

Bakshi N, Sinha C et al
Shared Decision Making: Decision Aid May Empower Patients

- Providing high quality information about options
- Informing about associated risks and benefits
- Help clarify and identify their values
- Clarify and define their preferences
- Empower them to share in the process of informed medical decision making
Decision Aid: Ottawa Decision Support

**Decisional Needs**
- Decisional conflict (uncertainty)
- Knowledge & expectations
- Values
- Support & resources
- Decision: type, timing, stage, leaning
- Personal / clinical characteristics

**Decision Quality**
- Informed
- Values-based

**Actions**
- Delay, continuance

**Impact**
- Values-based health outcomes
- Regret & blame
- Appropriate use & costs of services

**Decision Support**
- Clarify decision & needs
- Provide facts, probabilities
- Clarify values
- Guide in deliberation & communication
- Monitor / facilitate progress

**Counseling**

**Decision Tools**

**Coaching**
Decisional Needs Assessment & Development of Decision aid

• A nationwide sample (n=205) of patients, caregivers, stakeholders and health care providers
• Semi-structured qualitative interviews conducted, recorded, transcribed.
• Transcripts coded using QSR NVivo 10.
• A web based decision aid developed
• Two iterative cycles of beta testing (111 subjects)
• Decision Aid upgraded based on stakeholder and peer feedback
Community partnership in Needs Assessment & Decision Aid Development

Largest Direct Engagement of sickle cell stakeholders (Over 1000 individuals)

Patients

CBOs

Families

Healthcare providers

Policy Makers
Decisional Needs: What Did We Learn

- Patients do not hear about long term risks of SCD such as organ damage or death
- Patients are unaware of BMT
- Patients and caregivers prefer doctors to have an open conversation, provide all options
- Patients and caregivers want to hear the experience of others with medications and treatments

Ross D, Sinha C, Bakshi N 2016
Design of Decision Aid: What did Stakeholders Teach Us?

High quality unbiased & outcomes based information

Pros and cons of treatment options

Interactive user friendly environment

Stylistically pleasing lots of visual content

Easy to read plain language

video testimonials from individuals and families
• Provides disease-related information
• Assists to identify what is important to you, preferences and how to talk with health care providers
• Options for SCD treatment
• Treatment risks, benefits, outcomes
Steps to Making an Informed Decision

Decision Making Steps
- Sickle cell disease and supportive care
- Treatment options for sickle cell disease
- What is Important to Me
- Making a Decision with my doctor
- Evaluate Decision
Sickle Cell Disease and Supportive Care

What would you like to review first?

- Heredity
- Newborn Screening
- Birth Control & Pregnancy
- Sickle Cell Disease & Usual
- Psychosocial Care
- Caring for Myself at Home
Treatment Options for Sickle Cell Disease

You may consider treatment:

- In addition to managing and monitoring your sickle cell

What is Important to Me:
- Learn about the treatment option you are thinking about
- As you learn, think about what is important to you
- Answer the questions in the purple boxes
- What is Important
- Review what you feel is important in the section: What is Important to Me?
Discussing Hydroxyurea

Let's talk about Hydroxyurea

Treatment options for sickle cell disease

Chronic Blood Transfusions

Hydroxyurea

Let's talk about Hydroxyurea

How does Hydroxyurea work?

What are the risks and benefits?

Who should consider Hydroxyurea?

Taking Hydroxyurea

How will taking Hydroxyurea affect me?

Hydroxyurea Report

Bone Marrow Transplantation

Decision Making Steps
Making a decision with my doctor

Making a decision involves:

- Preparing for discussion
  - You have reviewed what is important to you
  - Talk with your family
  - Now review things you may want to discuss with your doctor
- Discussing treatment options
  - Plan to discuss your treatment with your doctor at a clinic
    - Take any important papers or notes to help you with your discussion
    - Consider who else you want to join in the discussion, such as your
      - Family
      - Friends
- Developing a plan of action
  - After you have made a decision you will develop a plan of care with your doctor
Evaluating my decision

Bone Marrow Transplantation – Prior to Transplant

- Health
- Pain
- Complications of Bone Marrow Transplant
- Fertility
- New illness or worse off
- Side Effects
- Psychological Health
- Managing your Health
- Family Life
- Social Life
- Physical Appearance (how you look)
- Your Future

Talk to your doctor about your sickle cell disease health and your treatment
Video Library

sickleoptions.org/en_US/video-library/

Questions?
Contact:
Diana.Ross@emory.edu
The SHARE Approach

5 Essential Steps of Shared Decision Making

1. Seek your patient's participation.
2. Help your patient explore & compare treatment options.
3. Assess your patient's values & preferences.
4. Reach a decision with your patient.
5. Evaluate your patient's decision.

Strategies to Promote Shared Decision Making for HU in SCD

- Invite all stakeholders in the family to attend clinic visit
- Share trusted educational resources from Internet
- Address safety concerns
- Initiate discussion about hydroxyurea early after diagnosis on NBS with plan to initiate HU at 9 months.
- Plan on multiple follow-up conversations
- Share written protocol for follow-up

Checklist for Shared Decision Making

✓ Healthcare decision regarding SCD identified
✓ More than one way to manage SCD described
✓ Different sources of information offered
  (Brochure/website/other patients)
✓ Different options including possibility of doing nothing discussed
✓ Pros/Cons/possible outcomes of options discussed
✓ Ideas expectations about managing SCD discussed

Checklist for Shared Decision Making (Cont’d)

- Concerns/worries about managing SCD discussed
- Ensure information understood
- Opportunities to ask questions
- Preference to take part in the decision/not is respected
- Decision was made/postponed
- The possibility of returning to decision discussed

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CE-12-11-4318 Krishnamurti (PI)
Patient Centered Outcome Research Institute
Programs and Resources for Health Professionals and Patients

Lensa Idossa, MPH
Program Analyst, Diverse and Medically Underserved Populations
Patient Navigation & Advocacy
National Marrow Donor Program/Be The Match
Jason Carter Clinical Trials Program
To help patients with blood disorders and immune systems diseases find and join clinical trials

• **One-on-one support** for patients & families to help answers questions and guide their clinical trials search

• **Online search tool**: [JasonCarterClinicalTrialsProgram.org](http://JasonCarterClinicalTrialsProgram.org)

• **Easy-to-understand resources** to learn about treatments and clinical trials

**Contact:** Scott Kerwin, RN, MN, CCRC, CCRN
Clinical Trial Patient Education Specialist

**Phone:** 1 (888) 814-8610

**Email:** [clinicaltrials@jcctp.org](mailto:clinicaltrials@jcctp.org)
Sickle Cell Disease Resource Center

• Visit BeTheMatchClinical.org/CureSCD to find:
  – Educational activities
  – The latest sickle cell disease (SCD) treatment guidelines and choices,
  – BMT CTN trials for SCD
  – Resource materials for clinicians, patients and families
    • SCD Booth in a Box (pictured)
HCT Quick Reference Guidelines

2018 Clinical Guidelines include:

- HCT referral guide for autologous and allogeneic transplant for 20+ diseases
- Recommended post-transplant screening, preventive practices, and vaccination schedules
- Clinical screening and prognostic tools for early detection of chronic GVHD, with photo atlas

Available in mobile app, print and online: BeTheMatchClinical.org/guidelines
Be The Match Patient Support Center

Our services include:

• Confidential telephone counseling and one-on-one support for your patients and families
• Financial grants for patients
• Support groups and telephone workshops
• Caregiver support
• Information and support in many languages
• Educational books, DVDs, newsletters and fact sheets

Order, view or download: BeTheMatchClinical.org/order

Bilan, MSW, BMT Patient Navigator

Phone: 1 (888) 999-6743
Email: patientinfo@nmdp.org
Giving New Voice
An SCDAA Overview.....

“Break The Sickle Cycle”

By: Beverley Francis-Gibson
President & CEO
Sickle Cell Disease Association of America, Inc.
Mission

"To advocate for people affected by sickle cell conditions and empower community-based organizations to maximize quality of life and raise public consciousness while advancing the search for a universal cure."
Serving the Community

- Forty-five (45) community-based organizations (CBOs) designed to serve individuals and families in their respective communities by offering program and outreach services
- Assist clients with finding quality healthcare
- Maintain databases of individuals and families that have been served over the years
- Community Outreach & education
Get Connected
Patient-Powered Registry

- Establish a network of children, adults and families living with sickle cell disease, SCDAA member organizations, health care providers and other community-based organizations to distribute information related to clinical care, research, health services, health policy and health care advocacy
- More than 6,500 individuals have signed-up
- Visit: www.GetConnectedSCD.com
SCDAA will ensure the success of partnerships by ….

- Incorporating patient and family involvement at all levels
- Keeping the SCD Community informed
- Ensuring communication & transparency
- Utilizing CBO’s for patient education & access
- Providing partnership exposure in the Community
SICKLE CELL COMMUNITY CONSORTIUM

Purpose

To provide a platform for patients/caregivers/CBOs to become directly involved in defining problems/needs/gaps in the sickle cell community and identify strategies to address those problems/needs/gaps.
Clinical Trial Related Programs

Patient-Centered Outcomes Initiative (PCOI) - Developed to involve patients and caregivers in identifying the research topics, study endpoints and alternative/surrogate markers that matter to the patient.

- COMPASS: Community Participation to Advance the Sickle Cell Story: capturing the patient voice to determine research priorities
- Clinical Trials Initiative - launched as part of PCOI to increase participation in clinical trials

Warrior University - An online webinar series providing curriculum-based education on a wide variety of topics, including clinical trials

Visit: [http://sicklecellconsortium.org/](http://sicklecellconsortium.org/)
ASH Sickle Cell Disease Coalition

• To amplify the voice of the sickle cell disease stakeholder community to improve outcomes for individuals with SCD.

• Priorities:
  – Access to care in U.S
  – Training and professional education
  – Research and clinical trials
  – Global issues

Thank you
Stay Informed

• Free resources to support decision-making and education
  – BeTheMatchClinical.org/order

• Free clinical education (CE) courses and events
  – BeTheMatchClinical.org/education

• Subscribe to Resource Connection for Health Professionals
  – BeTheMatchClinical.org/enews