Welcome!

TO THE CURE SICKLE CELL INITIATIVE WEBINAR

Dr. Traci Mondoro
Chief of Translational Blood Science and Resources Branch at NHLBI, Division of Blood Diseases and Resources
Welcome from Dr. Traci Mondoro

➢ Background of The Cure Sickle Cell Initiative
  • The Cure Sickle Cell Initiative (CureSCi) is a program of the National Heart, Lung, and Blood Institute, which is part of the National Institutes of Health. CureSCi is a collaborative, patient-focused research effort designed to accelerate promising genetic therapies to cure sickle cell disease.

➢ Presenters
  • Heather Avant, CureSCi Community Input Panel Chair
  • Dr. Marjorie Deloie-Brewer, Mental Health & Wellness Initiative for the Sickle Cell Consortium, Medical Director, Sickle Cell Association of Delaware
  • Beverly Francis-Gibson, President and CEO of SCDA
  • Dr. Steven Hardy, Director of Psychology and Patient Care Services, Center for Cancer and Blood Disorders at Children’s National Hospital
  • Panelists: Clayton Andrews, Maria Rivera, Dr. Soumitri Sil, and Teonna Woolford
    • Moderator: Dr. Victoria Coleman-Cowger, Clinical Psychologist and Senior Project Leader – Scientist at Emmes (CureSCi Coordinating Center)

➢ Housekeeping Items
  • We will try to adhere to our schedule so that there is enough time for questions.
  • All attendees will be placed on mute during speaker presentations.
  • Today’s webinar includes a Q&A session which will be conducted as part of our panel discussion.
  • You may submit a written question using the Q&A feature located on the bottom of the screen at any point during the presentations.
  • If your written question is directed to a specific speaker, please indicate that in your question, if possible. We will make every effort to get to your question during the webinar but if we are unable to respond due to time constraints, we will follow up with you via the email provided during your registration.
  • Please do not use the ‘Chat’ feature to submit questions.
Community Input Panel

Perspectives on Mental Health and the Needs of the SCD Community

Heather Avant
Community Input Panel - Chair
Sickle Cell Patient Advocate
**Managing Hope**

- Curative Therapies offer new hope to many patients living with sickle cell disease.
  - Hope is the foundation to any type of recovery, the idea that the patient wants to be healed and cured offers a new concept of life that has not been offered in the past.

**Communication**

- Providers must ensure patients and caregivers have a complete understanding of the curative therapy process.
- It is imperative that providers prioritize the mental health of patients throughout the curative therapy process.

**Community**

- Potential change of locations
- Loss of community
- Loss of job and income
- Effect on caregivers
- Adapting to new surroundings
Managing Expectations

Conditioning / Transplant

- Offering full disclosure and giving participants time to do a risk benefit analysis.
  - What does each decision mean and how does each part of the process effect the patient emotionally?

- Providers need an upfront and honest offering strong doses of truth regarding the conditioning process and what is involved in the transplant process. While patients are hospitalized and at home.

- Understanding disease progression of sickle cell.
  - Some aspects of specialized care will continue and be expected after transplant.

- Acceptance of needing a transplant.

- What the patient can expect in 1, 5, 10 and 15 year mark.
Managing Emotions

**Adverse Effects**
- Sterilization
- Secondary Cancer
- PTSD
- Transplant Failure
- Death

**Identity Issues**
- Loss of self and identity as a sickle cell patient
- Stigma within the healthcare system
- Dealing with anxiety and depression

**Survivor’s Guilt**
- Thriving after loss due to SCD
- Watching SCD Friends and Family still struggle with their medical issues
- Caregivers, friends and family may have survivor’s guilt
Resources

➢ MALE

➢ FEMALE
   1. http://providers.therapyforblackgirls.com

➢ LGBTQIA+
   1. https://www.nqttcn.com/

➢ GENERAL
Advocacy Leadership Insight on Mental Health and SCD

Dr. Marjorie DeJoie-Brewer & Beverley Francis-Gibson
Mental Health & Psychosocial Considerations for Gene Therapy

Insight on Mental Health & Sickle Cell Disease

Sickle Cell Community Consortium

Dr. Marjorie DeJoie-Brewer
Mental Health & Wellness Initiative, Sickle Cell Consortium
Medical Director, Sickle Cell Association of Delaware
SC3: Sickle Cell Mental Health & Wellness Initiative

- Sickle cell patients, caregivers, and CBO partners prioritized a need to increase awareness and de-stigmatization, and support for mental health issues and concerns (2016).

- Mental Health Track added to annual Warriors Convention and launched Mental Health Initiative with monthly presentations and “Community Conversations”.

- Recognized need to additional mental health support specifically with curative therapies. Formed partnerships with S.T.A.R. and Be The Match to create enduring materials on curative therapies.

- Mental Health Initiative expands to include Mental Health & Wellness (2020).

Mental Health & Psychosocial Considerations

Formal webinars with psychologists and mental health providers

Small group chats and moderated discussion

Partnered with Be The Match to offer one-on-one counseling support for Warriors

Warrior Games & Other Activities

- Slide into the DMs: Icebreaker
- Online Murder Mystery: Team-based
- Digital Escape Room: Team-based
- Read-A-Thon
- Online Scavenger Hunt
- Short Story Competition
- Creative Content Creators
Curative Therapies: The Good, The Bad, The Ugly

- Patients & Caregivers express concerns with perceived lack of transparency and support.
- Patients seeking curative therapies outside of on-campus NIH trials expressed lack of support for mental health concerns.
- Patients outside of NIH campus were offered little to no fertility support. Loss of fertility is a major effector of overall mental health.
- Patients unaware of instances where mental health resources were available. No information provided on how to access to mental health services.
- Fear of life without sickle cell disease. Concerns with lack of continuing support. “Am I still a warrior?”
- Caregivers experience little to no mental health support. Constant worry. “Am I doing the right thing?” “Do I have the right to make this decision for my child?”
- Caregivers worry about not only overall health, but how their child will “change”.
Mental Health & Psychosocial Considerations
Mental Health & Wellness Initiative has expanded to include an added focus on the mental health challenges related to curative therapies in sickle cell disease.

Ongoing & New Plans include:
- Community Presentations, Community Conversations, Panels, and Roundtables
- Evening Wholeness: Yoga, Breathing, Mindfulness, and Mental Health Checks
- Curative Therapy Town Halls & Warrior University series
- Vitamin SC3 Podcast to include monthly mental health discussions

Resources:
- Mental Health Sickle Cell Classroom – archive of videos of community discussions for patients and caregivers.
- Mental Health & Wellness Website – repository of mental health resources by group and demographic.
Advocacy Leadership Insight to Mental Health and Sickle Cell

Beverley Francis-Gibson
President and CEO
Sickle Cell Disease Association of America
The Sickle Cell Disease Association of America, Inc. (SCDAA) serves as the nation’s only volunteer organization working full time on a national level to resolve issues surrounding sickle cell disease.
"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."
OUR MEMBERS

➢ Fifty-three (53) community-based organizations 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
➢ Educate local communities
➢ Support Groups
➢ Summer Camps
Mental Health and SCD

- Complications of living with sickle cell disease can cause a large emotional burden
- Pain, fatigue and sleep disturbance are linked to depression
- About 1 in 3 people living with SCD experience depression
- Anxiety and isolation; low self-esteem are other symptoms
EXPERIENCES OF SICKLE CELL PATIENTS DURING COVID

- Mental Health: Fear, anxiety, depression, isolation, stress
- Coronavirus grief: loss of routine, control, security; grief
- Fear of hospitalization during COVID; sitting in pain for long periods
- Blood shortages—low blood supply for transfusions
- Economic; loss of income due to COVID-19; paying bills
- Exposure to COVID if they return to work (ie. service industry)
- Distance to services/care (patients traveling 4 hours by public transportation)
- Living in densely populated urban areas=increased risk
- Access to medication (pharmacies closed)
- Fear and concern about COVID-19 vaccine
Benefits of Getting Help

- Overcome fears and insecurities
- Cope with stress
- Incorporate self-care methods into your lifestyle
- Improve your relationships with friends, family, co-workers
- Identify symptoms before they get worse
- Get healthier with diet and exercise
UNSTOPPABLE:
WORKING TOGETHER FOR SICKLE CELL

Join us for our 49th Annual National Convention.
October 12-16, 2021 | Virtual
bit.ly/NationalConvention2021
#SCDUNSTOPPABLE
Grief and SCD: youth, caregiver
Fertility and Pregnancy
Bone Marrow Transplant
Mental Health and SCD
Gene Therapy
Pain Management
Sickle Cell and Access to Reproductive Health Services: An Advocate’s Perspective
Patient Readiness and Resilience Working Group

Steven Hardy, Ph.D.
Chair, CureSci Patient Readiness and Resilience Working Group
Director, Psychology & Patient Care Services, CCBD, Children’s National Hospital
Assistant Professor, Psychiatry & Behavioral Sciences, George Washington University SOM
Recognition that:

Deciding to participate or not participate in a clinical trial for genetic therapy is not simple.

- I’m feeling okay now, should I take a risk?
- Is it safe?
- My family doesn’t agree with my decision, what should I do?
- Will I be able to have kids?
- If it works, am I still a Warrior?
Origins

Recognition that:

Psychosocial factors will influence a person’s readiness for genetic therapy and therapy outcomes will affect a person’s psychosocial well-being in the short-term and long-term.
Origins

Is the patient ready?

What supports will be needed?

How to ensure it’s safe and ethical?

Gynecology
Cardiology
Pulmonology
Neurology
Nephrology
Radiology
Hematology
Urology

Genetic Therapy

Cultural
Social
Psychological

Spiritual


CURE SICKLE CELL.

NIH

National Heart, Lung, and Blood Institute
Purpose and Activities

**Purpose of the PRR WG**

- Bring together a group with expertise in psychological aspects of SCD and engage with the patient community to identify and prioritize key psychosocial issues that should be considered when conducting genetic therapy clinical trials.

**Activities**

- Identify and raise awareness regarding key psychosocial areas relevant to genetic therapy in SCD.
- Draft best practices for assessing trial readiness, reducing potential for harm, and promoting positive psychosocial outcomes following genetic therapy.
- Lend support the development of funding opportunities and educational materials related to psychosocial issues in genetic therapy for SCD.
PATIENT READINESS AND RESILIENCE WORKING GROUP

Members

Clayton Andrews, MS, LPC
SC MAN
Sickle Cell Mental Health and Wellness Initiative

Shawn Bediako, PhD
University of Maryland, Baltimore County

Lori Crosby, PsyD
Cincinnati Children’s Hospital Medical Center

Steven Hardy, PhD
Children’s National Hospital

Charles Jonassaint, PhD, MHS
University of Pittsburgh

Jerlym Porter, PhD, MPH
St. Jude Children’s Research Hospital

Maria Rivera
Sickle Cell Consortium

Soumitri Sil, PhD
Children’s Healthcare of Atlanta

Cecelia Valrie, PhD
Virginia Commonwealth University
Accomplishments to Date

Progress

➢ Monthly videoconference meetings, first meeting held September 28, 2020
➢ Soliciting input from patients, community organizations, and other stakeholders regarding psychosocial considerations for genetic therapy trials
➢ Reviewing existing scientific literature, related professional documents, and similar models developed by other groups for different patient populations
➢ Identifying initial guiding questions and framework for development of best practice statements
➢ Multiple small group writing meetings
➢ Expanded membership in June 2021
Accomplishments to Date

Guidance from patients, advocates, stakeholders

- Clear information and transparency is needed about possible risks and consequences
- Investigators need to help foster realistic expectations
- It could be helpful to provide therapy or counseling before the trial – signing informed consent does not mean someone is fully prepared to participate
- A patient’s history of mental health concerns needs to be considered
- There needs to be a focus on mental health throughout the course of the clinical trial (before, during, after)
- There are often external social stressors that need to be considered and addressed
Accomplishments to Date

Guidance from patients, advocates, stakeholders

- Education about the trial should come from the PI but supported by other members of the team
- Social workers are vital members of the team across the course of treatment, but some institutions may not have these resources
- It is important to engage the entire family, especially with younger patients
- It is important to proactively address barriers and mistrust
- Patients need educational materials that are not sponsored by industry
- There is a need for indefinite follow-up because some patients need to be monitored closely, some will continue to have symptoms, and life priorities and associated challenges may change over time
Accomplishments to Date

Review of literature, professional documents, models

➢ Many individuals with SCD say they have limited knowledge about gene therapy & worry about chemotherapy & risks (cancer, hair loss, infertility)

➢ Older patients report being more likely to consider gene therapy compared to younger patients

➢ One study reported that primary caregivers of children with SCD viewed gene therapy as a potentially less risky treatment option compared to HSCT


Accomplishments to Date

Review of literature, professional documents, models

- Very limited scientific literature on psychosocial outcomes following genetic therapy for SCD

- Initial data suggest improvement in aspects of health-related quality of life following gene therapy:
  - Reduced pain intensity, pain interference, anxiety, depressive symptoms, and fatigue
  - Improved satisfaction with social roles and physical function


Accomplishments to Date

Review of literature, professional documents, models

Conclusion 7-4

➢ Stem cell transplant carries a high psychosocial impact. Holistic care that addresses the psychological, economic, social and spiritual impact of transplantation is critical and should become the standard of care from the pre-transplantation period to the post-transplant long-term follow-up stage.

Conclusion 7-5

➢ Gene therapy advances and clinical trials are proceeding at a fast pace. Education and patient-facing materials on the risks and benefits of gene therapy are urgently needed, as is research to elucidate the long-term implications of gene therapy.

Accomplishments to Date

Review of literature, professional documents, models
Guiding Question

If a person with SCD chooses to pursue a genetic therapy trial, what level of psychosocial support should be provided by an investigative team & institution to minimize psychosocial harm and optimize benefits?
Guiding Question

If a person with SCD chooses to pursue a genetic therapy trial, what level of psychosocial support should be provided by an investigative team & institution to minimize psychosocial harm and optimize benefits?

Best practices for assessing knowledge and readiness for gene therapy or gene editing clinical trials

Best practices for assessing psychosocial risk and protective factors in the context of gene therapy or gene editing clinical trials
• Primarily a discussion/clinical interview, though cognitive testing and questionnaires could support the assessment.

• Should be viewed as informational for both patients and investigative teams, used to guide delivery of supports to enhance patient readiness.

• Should not be used to pressure or persuade, nor should its findings be used to permanently exclude anyone from receiving a potentially therapeutic treatment if desired.
Gather information about a patient’s understanding of and perceived readiness for genetic therapy.

Encourage open dialogue regarding concerns about genetic therapy.

Provide an overview of psychosocial factors likely to influence the clinical trial and affect relevant outcomes.

Identify patient strengths to be leveraged to promote psychosocial well-being before, during, & after the trial.

Identify risks to be considered & addressed through tailored education, psychosocial support, & community resources.
Future Plans and Recommendations

➢ Finalizing initial draft of Pre-Genetic Therapy Assessments of Patient Readiness and Resilience document

➢ Soliciting feedback from patients, caregivers, advocates, subcommittees, and other stakeholders

➢ Developing support documents for implementation of Pre-Genetic Therapy Assessment of Patient Readiness and Resilience

➢ Best practices for supporting patient resilience during and after trial

➢ PRR working group as a resource to others within CureSCi

➢ Looking for opportunities to enhance communication and cross-collaboration with other subcommittees and working groups
Acknowledgements

➢ PRR Working Group Members
➢ Community Input Panel and Research Coordinators Committee Members
➢ CureSCi Leadership
➢ The Emmes Company
Psychosocial Considerations for Gene Therapy Trials

PANEL DISCUSSION

Moderated by
Dr. Victoria Coleman-Cowger
Clinical Psychologist and Senior Project Leader
Scientist at Emmes (CureSCI Coordinating Center)
Remember to visit curesickle.org to learn more and sign up for updates!

Follow us on Twitter: @theCureSCi

YouTube channel: https://www.youtube.com/channel/UCTNbeEdOrlNdfbHR8qoEq5sA
Thanks for attending!