

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 DeJoie-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

1. <https://www.nimh.nih.gov/health/topics/men-and-mental-health/index.shtml>
2. Brother, you're on my mind: <https://www.nimhd.nih.gov/programs/edu-training/byomm/index.html>

➤ FEMALE

1. <http://providers.therapyforblackgirls.com>

➤ LGBTQIA+

1. <https://www.nqttcn.com/>

➤ GENERAL

1. <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC2594635/pdf/jnma00311-0027.pdf>
2. <https://www.ncbi.nlm.nih.gov/pubmed/18158366>
3. <https://bethematch.org/patients-and-families/life-after-transplant/coping-with-life-after-transplant/>

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

Mental Health & Psychosocial Considerations

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).
- “Curative Therapies: The Good, The Bad, The Ugly” Community Conversation at the 8th Annual Sickle Cell Warriors Convention.

Mental Health & Psychosocial Considerations

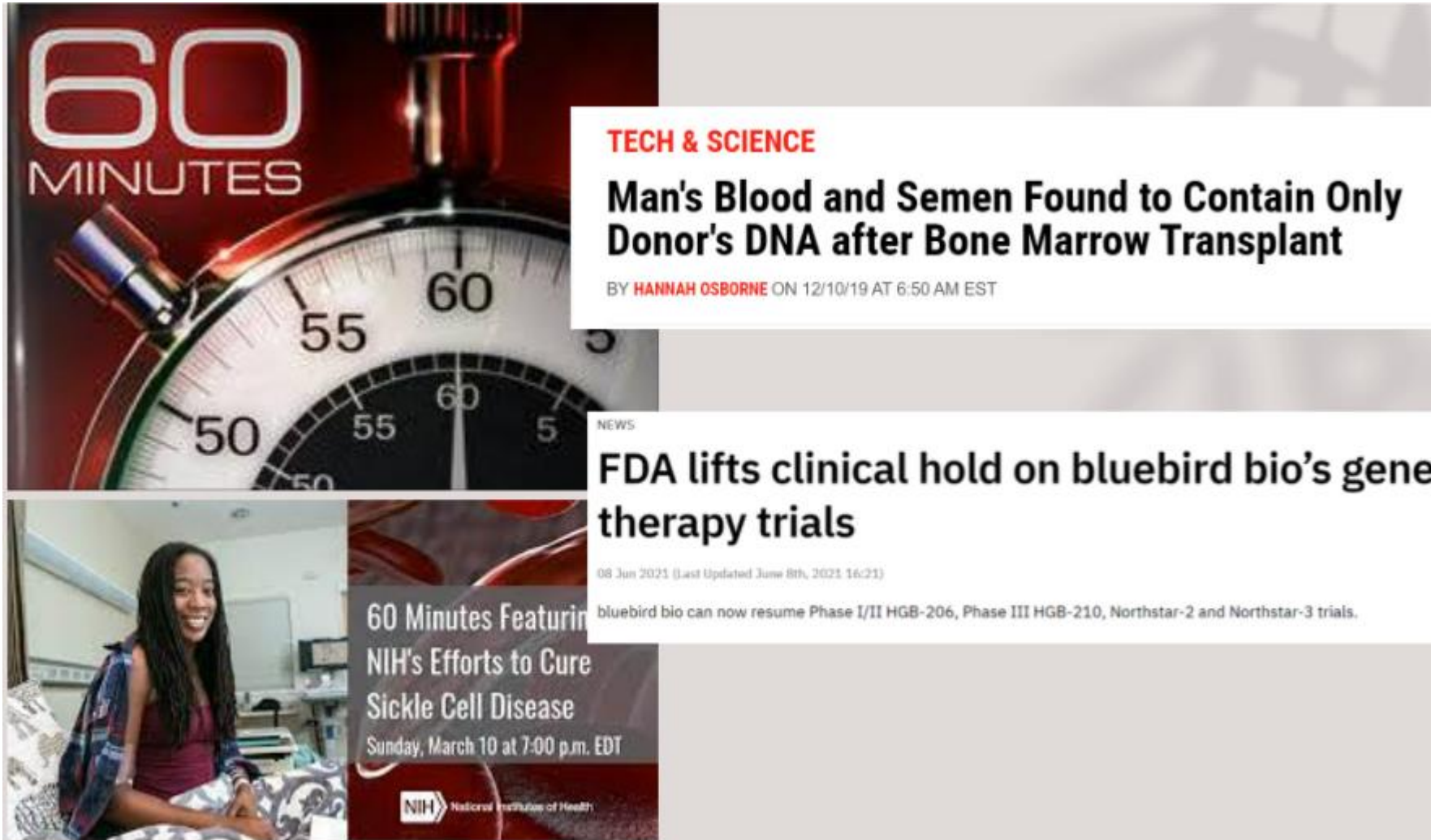


Mental Health & Psychosocial Considerations

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



60 MINUTES

TECH & SCIENCE

Man's Blood and Semen Found to Contain Only Donor's DNA after Bone Marrow Transplant

BY **HANNAH OSBORNE** ON 12/10/19 AT 6:50 AM EST

NEWS

FDA lifts clinical hold on bluebird bio's gene therapy trials

08 Jun 2021 (Last Updated June 8th, 2021 16:21)

bluebird bio can now resume Phase I/II HGB-206, Phase III HGB-210, Northstar-2 and Northstar-3 trials.

60 Minutes Featuring
NIH's Efforts to Cure Sickle Cell Disease
Sunday, March 10 at 7:00 p.m. EDT

NIH National Institutes of Health

Mental Health & Psychosocial Considerations

Looking Forward

- 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

**CURE
SICKLE
CELL.**



UNSTOPPABLE:

WORKING TOGETHER FOR SICKLE CELL

**Join us for our 49th Annual
National Convention.**

October 12-16, 2021 | Virtual

bit.ly/NationalConvention2021

#SCDUNSTOPPABLE



SCDAA CONVENTION PROGRAM HIGHLIGHTS

- 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

Origins

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?

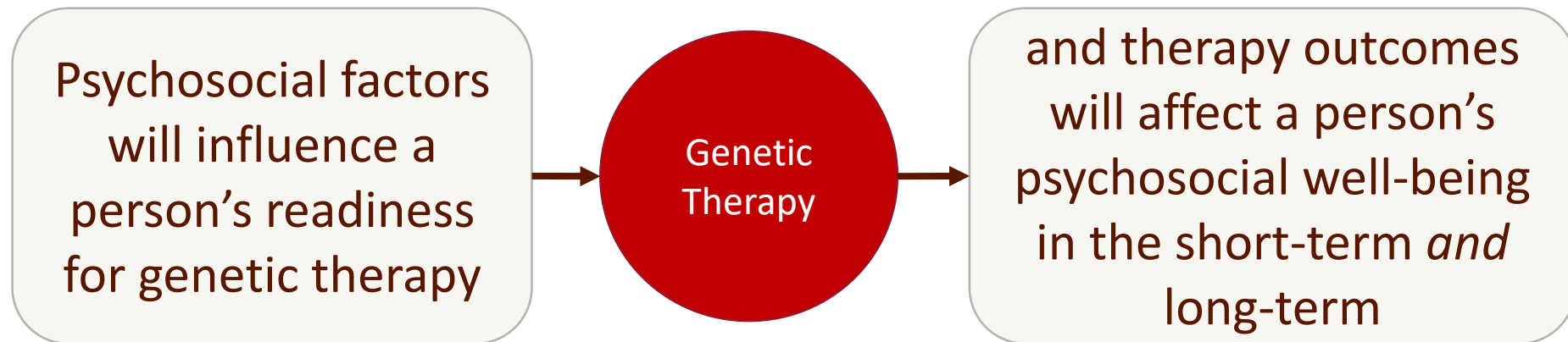
My family doesn't agree with my decision, what should I do?

Will I be able to have kids?

If it works, am I still a Warrior?

Origins

Recognition that:



Origins

Is the patient ready?



Gynecology

Nephrology

Cardiology

Radiology

Pulmonology

Hematology

Neurology

Urology

What supports will be needed?

Genetic
Therapy

How to ensure it's safe and ethical?



Cultural

Spiritual

Social

Psychological

Purpose and Activities

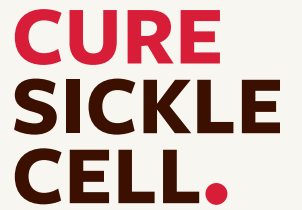
Purpose of the PRR WG

- 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

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

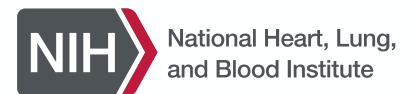
Sickle Cell Consortium

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

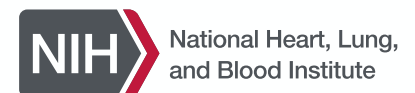
Review of literature, professional documents, models

- 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

Desine S, Hollister BM, Abdallah KE, Persaud A, Hull SC, Bonham VL. The Meaning of Informed Consent: Genome Editing Clinical Trials for Sickle Cell Disease. *AJOB Empir Bioeth*. 2020;11(4):195-207. doi:10.1080/23294515.2020.1818876

Strong H, Mitchell MJ, Goldstein-Leever A, Shook L, Malik P, Crosby LE. Patient Perspectives on Gene Transfer Therapy for Sickle Cell Disease [published correction appears in *Adv Ther*. 2017 Aug 2;:]. *Adv Ther*. 2017;34(8):2007-2021. doi:10.1007/s12325-017-0587-7

Sinha CB, Bakshi N, Ross D, Loewenstein G, Krishnamurti L. Primary caregiver decision-making in hematopoietic cell transplantation and gene therapy for sickle cell disease. *Pediatr Blood Cancer*. 2021;68(1):e28749. doi:10.1002/pbc.28749



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

Kanter J, Tisdale JF, Mapara MY, et al. Improvements in health-related quality of life for patients treated with LentiGlobin for sickle cell disease (bb1111) gene therapy [abstract]. *Blood*. 2020;136(suppl 1):10.

Thompson AA, Walters MC, Mapara MY, et al. Resolution of serious vaso-occlusive pain crises and reduction in patient-reported pain intensity: results from the ongoing phase 1/2 HGB-206 Group C Study of LentiGlobin for sickle cell disease (bb1111) gene therapy [abstract]. *Blood*. 2020;136(suppl 1):16-17.

Accomplishments to Date

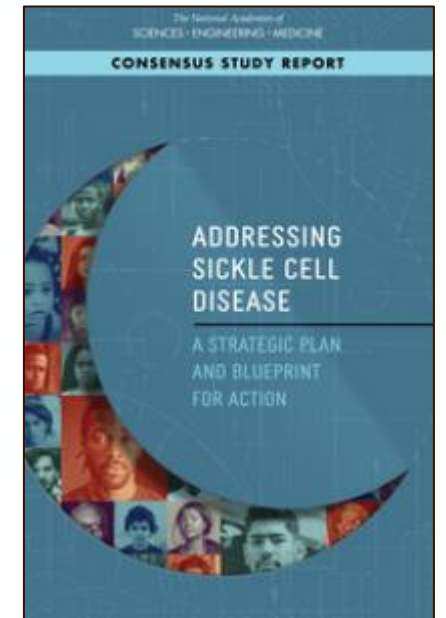
Review of literature, professional documents, models

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**.

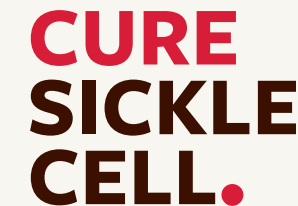
Conclusion 7-5

- 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.



National Academies of Sciences, Engineering, and Medicine. 2020. *Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action*. Washington, DC: The National Academies Press. <https://doi.org/10.17226/25632>.

Accomplishments to Date



Review of literature, professional documents, models



Surgery for Obesity and Related Diseases 12 (2016) 731–749

SURGERY FOR OBESITY AND RELATED DISEASES

ASMBS Guidelines/Statements

Recommendations for the presurgical psychosocial evaluation of bariatric surgery patients

Stephanie Sogg, Ph.D.^{a,b,*}, Jennifer Lauretti, Ph.D., A.B.P.P.^{c,d},
Lisa West-Smith, Ph.D., L.I.S.W.-S.^{e,f}

American Society for Metabolic & Bariatric Surgery

Published in final edited form as:

Thorac Surg Clin. 2012 November ; 22(4): 517–529. doi:10.1016/j.thorsurg.2012.08.001.

Psychosocial Issues Facing Lung Transplant Candidates, Recipients and Family Caregivers

Emily M. Rosenberger, BA^a, Mary Amanda Dew, PhD^{b,c,d,e,*}, Andrea F. DiMartini, MD^{b,f,g},
Annette J. DeVito Dabbs, PhD, RN^h, and Roger D. Yusen, MD, MPHⁱ

TRANSPLANTATION

Adverse psychological outcomes in long-term survivors of hematopoietic cell transplantation: a report from the Bone Marrow Transplant Survivor Study (BMTSS)

Can-Lan Sun,¹ Liton Francisco,¹ K. Scott Baker,² Daniel J. Weisdorf,³ Stephen J. Forman,⁴ and Smita Bhatia¹

0041-1337/01/7109-1189/0
TRANSPLANTATION
Copyright © 2001 by Lippincott Williams & Wilkins, Inc.

Vol. 71, 1189–1204, No. 9, May 15, 2001
Printed in U.S.A.

Transplantation[®] OVERVIEW

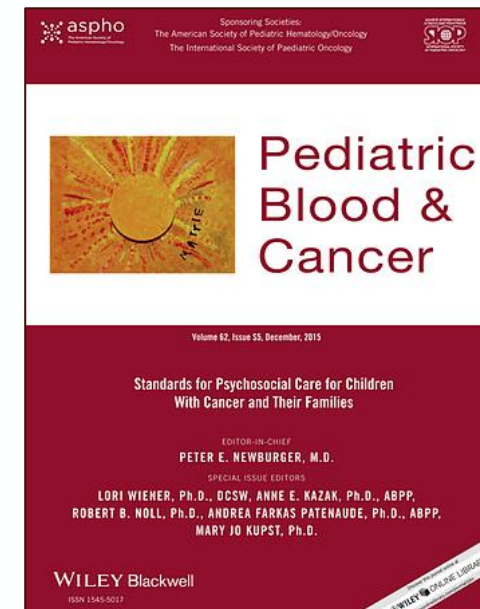
GUIDELINES FOR THE REFERRAL AND MANAGEMENT OF PATIENTS ELIGIBLE FOR SOLID ORGAN TRANSPLANTATION

THEODORE I. STEINMAN,¹ BRYAN N. BECKER, ADAANI E. FROST, KIM M. OLTHOFF, FRANK W. SMART,
WADI N. SUKI, AND ALAN H. WILKINSON

American Society of Transplantation

PSYCHOSOCIAL EVALUATION AND PREPARATION OF THE PATIENT

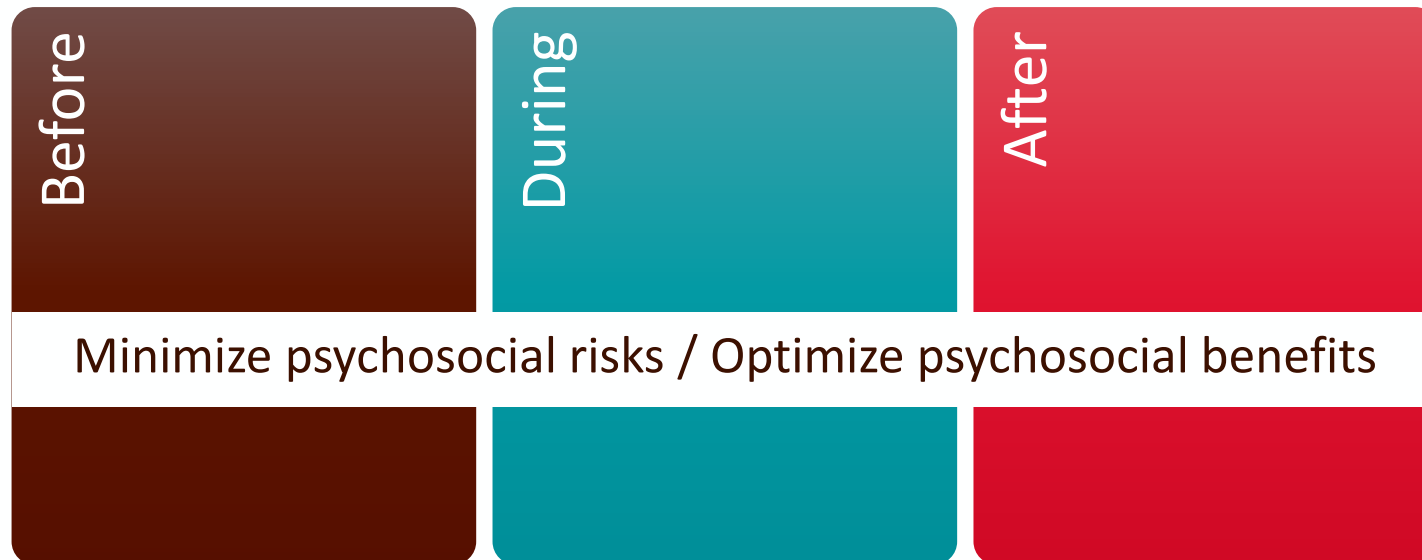
Not only is it possible that medical care could be improved with early referral, but there is better psychological adaptation for the patient awaiting a transplant when the entire spectrum of issues is approached (1). The psychosocial issues surrounding transplantation affect the ultimate success of a transplant, and recent studies suggest that the more positive



Current Activities

Guiding Question

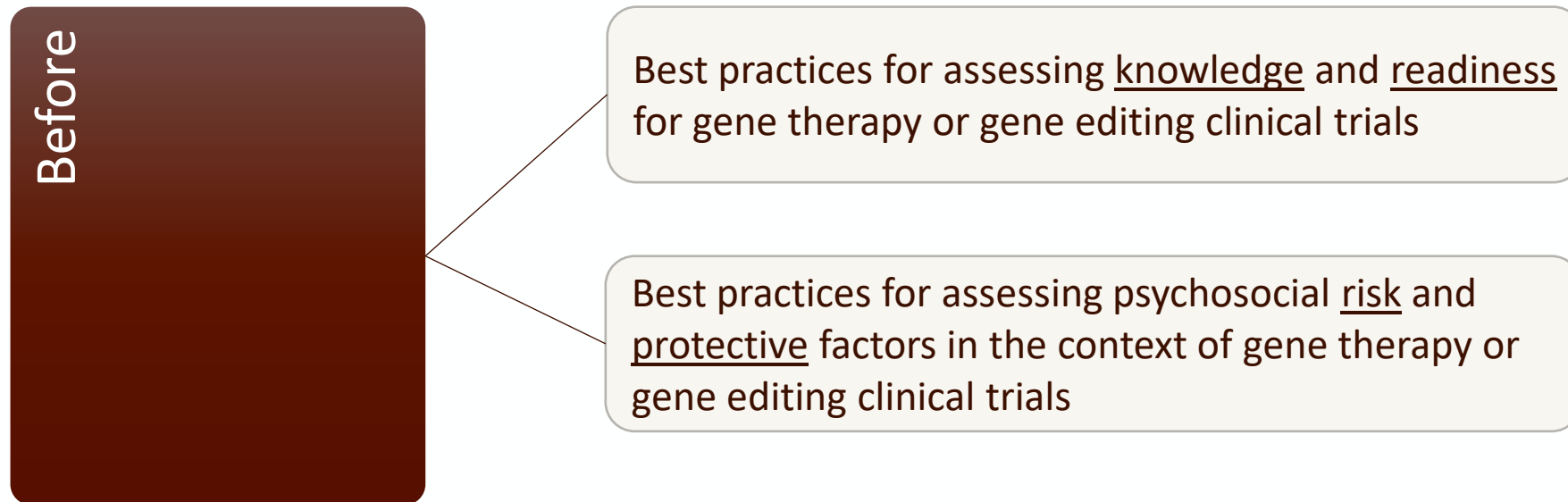
If a person with SCD chooses to pursue a genetic therapy trial, what level of psychosocial support should be provided by an investigative team & institution to minimize psychosocial harm and optimize benefits?



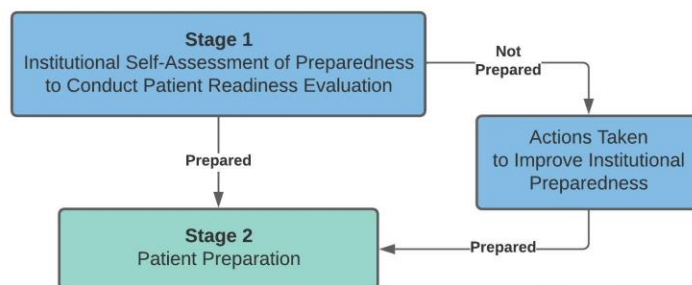
Current Activities

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?

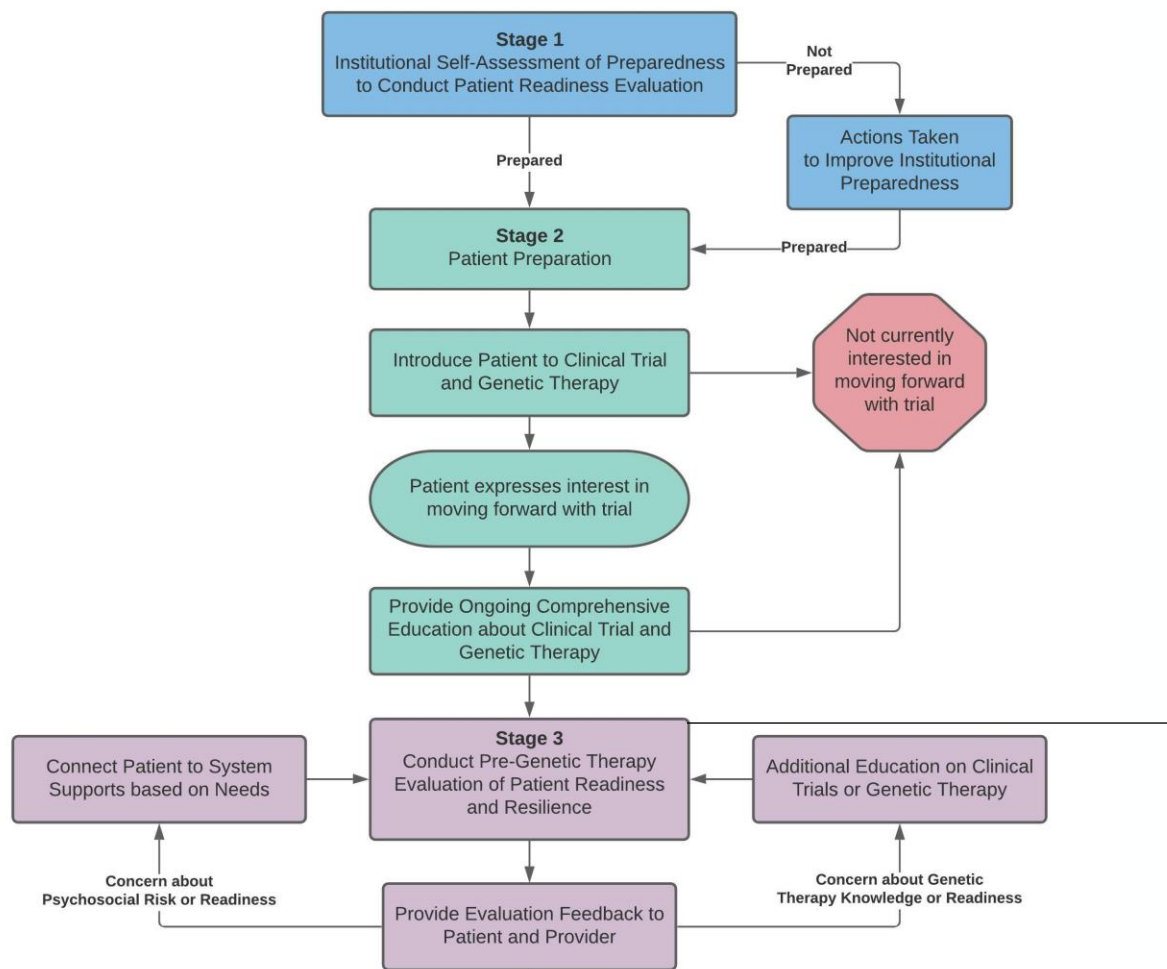


Draft Model for Patient Readiness and Resilience Evaluation



- 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.

Draft Model for Patient Readiness and Resilience Evaluation



- 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](https://twitter.com/theCureSci)

YouTube channel:

<https://www.youtube.com/channel/UCTNbeEdOrINdfbHR8qoEqsA>

Thanks for attending!

