



MEDICAL AND RESEARCH ADVISORY COMMITTEE (MARAC)

SCDAA MARAC Statement on Gene Therapy and Bone Marrow/Hematopoietic Stem Cell Transplantation in Sickle Cell Disease (Bone Marrow Therapies)

May 15, 2021 - The Sickle Cell Disease Association of America's Medical and Research Advisory Committee is aware of two cases of leukemia or other cancer-like conditions in people who have undergone gene therapy for sickle cell disease. This is more than would be expected given the small number of individuals who have undergone gene therapy to date. It is unclear why cancer developed in these individuals. The mission of SCDAA is to promote the best interests of individuals living with SCD. Accordingly, SCDAA MARAC urges action in three directions:

1. Informing patients, families and others about the current understanding of cancer risk as it pertains to sickle cell disease in a clear and accurate way.

1. Educate individuals with SCD who are participating in gene therapy (gene editing as well as lentiviral gene transfer) and other stem cell transplants (bone marrow therapies). Ensure that the consent forms reflect the recent findings and potential risks of leukemia and MDS.
2. Educate the greater SCD community by providing cancer risk estimates in the general population of individuals with SCD treated with other types of therapies.
3. Enlist experts in cancer genetic risk communication (e.g., NCI) and SCD stakeholders to develop education about risks of cancer in SCD.

2. Funding for research to better understand and address cancer-related risks of gene therapy and stem cell transplant (bone marrow therapies) in sickle cell disease.

1. Understand whether sickle cell disease intrinsically involves an increased risk of cancer; and, if so, what causes the increased risk.
2. Identify people living with sickle cell disease who are at increased risk for the development of cancer as they undergo "bone marrow therapies."
3. Identify components of "bone marrow therapies" that increase risk of cancer.
4. Bring together scientific experts from the fields of SCD, cancer, stem cell transplant and gene therapy to answer these questions.

3. Funding for and creation of a way to collect information about cancer-related risks from all studies that involve "bone marrow therapies" for sickle cell disease globally.

SCDAA Medical and Research Advisory Committee Members

Miguel R. Abboud, MD

Professor of Pediatrics and Pediatric Hematology-
Oncology
Chairman
Department of Pediatrics and Adolescent Medicine
American University of Beirut, Lebanon

Biree Andemariam, MD

Vice Chair, Sickle Cell Disease Association
of America
Director, New England Sickle Cell Institute
Associate Professor of Medicine
University of Connecticut Health
Farmington, Connecticut

Shawn Bediako, PhD

Professor
Department of Psychology
University of Maryland Baltimore County
Baltimore, Maryland

Andrew Campbell, MD

Center for Cancer and Blood Disorders
Children's National Health System
Associate Professor of Pediatrics
George Washington University School of Medicine and
Health Sciences
Washington, DC

Raffaella Colombatti, MD, PhD

Physician Azienda Ospedaliera-Università di Padova
Department of Womens' and Child Health
Clinic of Pediatric Hematology Oncology
Via Giustiniani 3 35129
Padova Italy

Lori Crosby, PsyD

Co-Director, Innovations in Community Research,
Division of Behavioral Medicine & Clinical Psychology
Co-Director, CCTST, Community Engagement Core
Psychologist, Research, Behavioral Medicine & Clinical
Psychologist
Cincinnati Children's
Professor, UC Department of Pediatrics
Cincinnati, Ohio

Deepika Darbari, MD

Center for Cancer and Blood Disorders
Children's National Health System
Professor of Pediatrics
George Washington University School of Medicine and
Health Sciences
Washington, DC

Payal Desai, MD

Associate Professor
Director of Sickle Cell Research
The Ohio State University
JamesCare at Ohio State East Hospital
Columbus, Ohio

James Eckman, MD

Professor Emeritus, Hematology & Medical Oncology
Emory University School of Medicine
Department of Hematology and Medical Oncology
Atlanta, Georgia

Mark Gladwin, MD

Professor and Chair
Department of Medicine
Founder, Pittsburgh Heart, Lung, and Blood Vascular
Medicine Institute
University of Pittsburgh
Pittsburgh, Pennsylvania

Jo Howard, MB Bchir, MRCP, FRCPath

Head of Red Cell/Sickle Cell Service
Guy's and St Thomas' NHS Foundation Trust
London, United Kingdom

Lewis Hsu, MD, PhD

Co-Chair, Medical and Research Advisory Committee,
Sickle Cell Disease Association of America
Vice Chief Medical Officer, Sickle Cell Disease
Association of America
Director of Pediatric Sickle Cell
Professor of Pediatric Hematology-Oncology
University of Illinois at Chicago
Chicago, Illinois

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Baba Inusa

Professor of Paediatric Haematology
Lead Consultant Paediatric Sick Cell and Thalassemia
Evelina London Children's Hospital
Guy's and St Thomas' NHS Foundation Trust
Women and Children's Academic Health
Faculty of Life Sciences and Medicine
King's College
London, United Kingdom

Elizabeth Klings, MD

Associate Professor of Medicine
Director, Center for Excellence in Sick Cell
Disease
Director, Pulmonary Hypertension Center
Boston University School of Medicine
Boston, Massachusetts

Lakshmanan Krishnamurti, MD

Professor of Pediatrics
Director of Bone Marrow Transplant
Joseph Kuechenmeister Aflac Field Force Chair
Aflac Cancer and Blood Disorders Center
Children's Healthcare of Atlanta/Emory University
Atlanta, Georgia

Sophie Lanzkron, MD

Director, Sick Cell Center for Adults
The Johns Hopkins Hospital
Baltimore, Maryland

Julie Makani, FRCP, PhD

Associate Professor
Department of Haematology and Blood Transfusion
Muhimbili University of Health and Allied Sciences
Dar es Salaam, Tanzania

Caterina P. Minniti, MD

Director, Sick Cell Center Montefiore Health System
Professor, Departments of Medicine and Pediatrics
Albert Einstein College of Medicine
Bronx, New York

Genice T. Nelson, DNP, APRN, ANP-BC

Program Director, New England Sick Cell Institute &
Connecticut Bleeding Disorders Programs,
UConn Health, Farmington, Connecticut
Board Member, Sick Cell Disease Association of America

Isaac Odame, MB ChB, MRCP(UK), FRCPPath, FRCPCH, FRCPC

Professor, Department of Paediatrics
University of Toronto
The Hospital for Sick Children Division of Haematology/
Oncology
Toronto, Ontario

Kwaku Ohene-Frempong, MD

Director Emeritus, Comprehensive Sick Cell Center
Emeritus Professor of Pediatrics, University of
Pennsylvania
President, Sick Cell Foundation of Ghana
Emeritus Board Member, Sick Cell Disease Association
of America

Gwendolyn Poles, DO

Former Medical Director, Kline Health Center
Faculty, Internal Medicine Program
UPMC Pinnacle
Harrisburg, Pennsylvania
Board Member, Sick Cell Disease Association of America

John D. Roberts, MD

Yale Adult Sick Cell Program
Smilow Cancer Hospital at Yale New Haven
New Haven, Connecticut

Wally Smith, MD

Professor
Scientific Director, VCU Center on Health
Disparities
Director, VCU Adult Sick Cell Program
Department of Internal Medicine Division of General
Internal Medicine
Virginia Commonwealth University
Richmond, Virginia

Crawford J. Strunk MD

Director, Sick Cell Disease and Hemoglobinopathy Clinic
Pediatric Hematology/Oncology Program
ProMedica Ebeid Children's Hospital
Toledo, Ohio

Immacolata Tartaglione, MD PhD

Department of Woman, Child and General and Specialist
Surgery
Università degli Studi della Campania "Luigi Vanvitelli"
Naples, Italy

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Marsha Treadwell, PhD

Director, Sickle Cell Care Coordination Initiative
Regional Director, Pacific Sickle Cell Regional
Collaborative
Professor of Psychiatry and Pediatrics
University of California San Francisco Benioff Children's
Hospital Oakland
Oakland, California

Winfred C. Wang, MD

Emeritus, St. Jude Faculty
Member, Department of Hematology
St. Jude Children's Research Hospital
Memphis, Tennessee

Russell E. Ware, MD, PhD

Director, Division of Hematology
Co-Director, Cancer and Blood Diseases Institute
Director, Global Health Center
Marjory J. Johnson Chair of Hematology Translational
Research
Cincinnati Children's
Professor, UC Department of Pediatrics
Cincinnati, Ohio

Julie Kanter Washko, MD

Associate Professor, Division of Hematology Oncology
Director, Adult Sickle Cell Clinic
University of Alabama at Birmingham
Birmingham, Alabama

Kim Smith-Whitley, MD

Professor of Pediatrics
Director, Comprehensive Sickle Cell Center
Clinical Director, Division of Hematology
The Children's Hospital of Philadelphia
Philadelphia, Pennsylvania
Board Member, Sickle Cell Disease Association of America

Wanda Whitten-Shurney, MD

CEO & Medical Director
Sickle Cell Disease Association, Michigan Chapter Inc.
Detroit, Michigan
Board Member, Sickle Cell Disease Association of America

Ahmar U. Zaidi, MD

Assistant Professor of Pediatrics
Comprehensive Sickle Cell Center, Children's Hospital
of Michigan
Director of Physician Network Development, University
Pediatricians
Wayne State University/Central Michigan University School
of Medicine
Detroit, Michigan