



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 three 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.**
 - 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.
 - Educate the greater SCD community by providing cancer risk estimates in the general population of individuals with SCD treated with other types of therapies.
 - 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.**
 - Understand whether sickle cell disease intrinsically involves an increased risk of cancer; and, if so, what causes the increased risk.
 - Identify people living with sickle cell disease who are at increased risk for the development of cancer as they undergo "bone marrow therapies."
 - Identify components of "bone marrow therapies" that increase risk of cancer.
 - 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.**

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