MARAC Statement: FDA Approval of First Gene Therapy for Children 2 Years and Older With Sickle Cell Disease

July 9, 2026 **-** The Medical Research and Advisory Committee (MARAC) of the Sickle Cell Disease Association of America, Inc., (SCDAA) is excited about the Food and Drug Administration's (FDA) approval of Casgevy (exagamglogene autotemcel) for sickle cell patients aged 2 years and older. We are encouraged to learn they are "committed to prioritizing and speeding up the review of products that address critical U.S. health priorities," including sickle cell disease (SCD).

Casgevy was previously approved for the treatment of patients aged 12 years and older with SCD. SCDAA has long advocated for a universal cure for sickle cell disease, and this approval by the FDA brings the sickle cell community much closer to realizing the goal of reducing the burden of living with SCD. Since the FDA approved two cell and gene therapies in 2023, SCDAA has worked with hematologists, member organizations, SCD community-based organizations, partners and stakeholders to educate families and patients about the potential benefits of cell and gene therapies.

It is well known that individuals with sickle cell disease can experience significant complications of the disease at very young ages, with some infants at risk of stroke and other end-organ damage. SCDAA's MARAC encourages parents of children diagnosed with SCD to discuss their child's care with a physician who is a sickle cell expert to evaluate the risks and benefits to their child and to determine if cell and gene therapy is the best option for their family. MARAC also advises parents and families to make themselves aware of the risk of infertility with these therapies and to pursue all available resources to ensure that their child has access to fertility services prior to undergoing any gene or cell therapy.

MARAC recognizes that while undergoing cell and gene therapies is an opportunity for individuals living with SCD to enjoy a life potentially free from the acute and chronic complications of the disease, there are ongoing risks and challenges to the procedure. Concerns relating to the manufacturing and collection of the cells and the length of time from the start of the gene therapy process to manufactured cell infusion can have a tremendous impact on patient health and family burden related to health and financial resources as well as any unknown long-term complications. Although these challenges should not prevent patients from pursuing the therapy, we encourage hematologists and gene therapy manufacturers to be forthcoming in informing patients and their families of these issues.

MARAC and SCDAA urge the FDA, gene therapy manufacturers and other groups crucial to the implementation of this very important treatment to continue to work with MARAC, sickle cell experts and the sickle cell community on issues such as the criteria to determine which patients are best suited for the therapy at such a young age, strategies for providing education and counseling to families considering the therapy for their young children and the importance of post-gene therapy follow-up and care which will be critical in this group. MARAC looks forward to continuing to partner with the pharmaceutical industry, government and the sickle cell community to find effective, safe and accessible treatments for the sickle cell population.