

ASH Guideline Recommendations for Sickle Cell Disease: Stem Cell Transplantation



What it covers

 Evidence-based guidelines to support individuals with sickle cell disease and their clinicians considering allogeneic stem cell transplants.



Why it matters

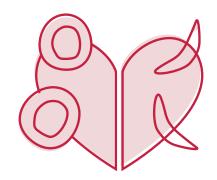
- SCD is a common inherited blood disorder in the United States. SCD results in significant health complications and affects quality of life.
- Allogeneic Hematopoietic stem cell transplant (HSCT), a process in which the individual's blood-forming stem cells are replaced with healthy cells from a donor (allogeneic), is currently the only potentially curative therapy for SCD.
- Guidelines are needed to inform how to apply HSCT in clinical practice, particularly to weigh the risks and benefits versus disease modifying/supportive therapies or potential curative therapies under development, such as gene therapy.



Who it affects

- Hematologists, internists, general practitioners, pediatricians, and other clinicians: Health care providers seeking clinical decision support to help identify which individuals with SCD should be considered for HSCT.
- Individuals with SCD: Individuals who may be discussing therapy options with their families and health care providers.
- Researchers: Those seeking to address potential gaps in evidence supporting treatment decisions.





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- HSCT should be considered over standard of care (transfusion) in individuals with SCD who have experienced a stroke or are at very high risk of stroke. Further, transplantation should be considered for all patients with neurologic injury who have a matched, related sibling donor. Recommendations point to evidence suggesting that children under age 13 who receive HSCT from a matched sibling donor have better outcomes than those older than age 13.
- For patients with frequent pain, as well as those with recurrent episodes of acute chest syndrome, the ASH guidelines suggest transplantation from a matched sibling donor over the standard of care.
- For individuals with an indication for HSCT who lack a matched sibling donor, the ASH guideline panel suggests transplantation from alternate donors only in the context of a clinical trial.
- In patients with an indication for transplant, the ASH guideline panel suggests transplantation with cells from a matched donor earlier in life due to the risk of irreversible SCD-related damage to the body that increases with age.

Total number of panel recommendations: 8 recommendations

REFERENCE

Kanter J, Liem RI, Bernaudin F, et al. American Society of Hermatology 2021 guidelines for sickle cell disease: stem cell transplantation. Blood Adv. 2021;5(18):3668-3689.

