

American Society of Hematology 2021 guidelines for sickle cell disease: stem cell transplantation.

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Blood Adv Blood Adv 3318 bloodadv 101698425 Blood Advances Blood Advances 2473-9529 2473-9537 The American Society of Hematology PMC8945587 PMC8945587.1 8945587 8945587 34581773 10.1182/bloodadvances.2021004394C 2021/ADV2021004394C 1 8 28 31 Clinical Guidelines American Society of Hematology 2021 guidelines for sickle cell disease: stem cell transplantation <https://orcid.org/0000-0001-7002-8891> Kanter Julie 1 Liem Robert I. 2 Bernaudin Françoise 3 4 Bolaños-Meade Javier 5 <https://orcid.org/0000-0002-5306-7167> Fitzhugh Courtney D. 6 <https://orcid.org/0000-0003-4439-7321> Hankins Jane S. 7 Murad M. Hassan 8 Panepinto Julie A. 9 Rondelli Damiano 10 Shenoy Shalini 11 Wagner John 12 Walters Mark C. 13 Woolford Teonna 14 <https://orcid.org/0000-0002-1333-5403> Meerpohl Joerg J. 15 16 <https://orcid.org/0000-0002-8535-9439> Tisdale John 6 1 Department of Hematology-Oncology, University of Alabama Birmingham, Birmingham, AL; 2 Division of Hematology, Oncology and Stem Cell Transplant, Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, IL; 3 French Referral Center for Sickle Cell Disease, Centre Hospitalier Intercommunal Créteil, Université Paris XII, Paris, France; 4 Société Française de Greffe de Moelle et de Thérapie Cellulaire, Lille, France; 5 Department of Oncology, Johns Hopkins University School of Medicine, Baltimore, MD; 6 Cellular and Molecular Therapeutics Branch, National Heart, Lung and Blood Institute, National Institutes of Health, Bethesda, MD; 7 Department of Hematology, St Jude Children's Research Hospital, Memphis, TN; 8 Division of Preventive Medicine, Mayo Clinic, Rochester, MN; 9 Medical College of Wisconsin/Children's Wisconsin, Pediatric Hematology, Milwaukee, WI; 10 Division of Hematology-Oncology, University of Illinois at Chicago, Chicago, IL; 11 St Louis Children's Hospital, Washington University School of Medicine, St Louis, MO; 12 Division of Blood and Marrow Transplantation, Department of Pediatrics, University of Minnesota, Minneapolis, MN; 13 Benioff Children's Hospital, University of California San Francisco, Oakland, CA; 14 Sickle Cell Reproductive Health Education Directive, Owings Mills, MD; 15 Institute for Evidence in Medicine, Medical Center, Faculty of Medicine, University of Freiburg, Freiburg, Germany; and 16 Cochrane Germany Foundation, Cochrane Germany, Freiburg, Germany Correspondence: Julie Kanter, University of Alabama at Birmingham, 1720 2nd St South, NP 2510, Birmingham, AL 35233; e-mail: jkanter@uabmc.edu . 28 9 2021 28 9 2021 5 18 403791 3668 3689 28 1 2021 23 6 2021 28 09 2021 29 03 2022 14 06 2026 © 2021 by The American Society of Hematology 2021 Abstract Background: Sickle cell disease (SCD) is a life-limiting inherited hemoglobinopathy that results in significant complications and affects quality of life. Hematopoietic stem cell transplantation (HSCT) is currently the only curative intervention for SCD; however, guidelines are needed to inform how to apply HSCT in clinical practice.

Objective: These evidence-based guidelines of the American Society of Hematology (ASH) are intended to support patients, clinicians, and health professionals in their decisions about HSCT for SCD.

Methods: The multidisciplinary guideline panel formed by ASH included 2 patient representatives and was balanced to minimize potential bias from conflicts of interest. The Mayo Evidence-Based Practice Research Program supported the guideline development process, including performing systematic evidence reviews (through 2019). The panel prioritized clinical questions and outcomes according to their importance for clinicians and patients. The panel used the Grading of Recommendations Assessment, Development and Evaluation (GRADE) approach, including GRADE Evidence-to-Decision frameworks, to assess evidence and make recommendations, which were subject to public comment.

Results: The panel agreed on 8 recommendations to help patients and providers assess how individuals with SCD should consider the timing and type of HSCT.

Conclusions: The evidence review yielded no randomized controlled clinical trials for HSCT in SCD; therefore, all recommendations are based on very low certainty in the evidence. Key recommendations include considering HSCT for those with neurologic injury or recurrent acute chest syndrome at an early age and to improve nonmyeloablative regimens. Future research should include the development of a robust SCD registry to serve as a comparator for HSCT studies.