Hematopoietic stem cell transplantation for hemoglobinopathies in adults

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ABSTRACT

Background: Hemoglobinopathies are the most prevalent monogenic disorders worldwide, and allogeneic hematopoietic stem cell transplantation (HSCT) remains the only established curative treatment. **Objective:** To provide an updated version of the Brazilian consensus published by the Brazilian Society of Cellular Therapy and Bone Marrow Transplantation, incorporating recent advances in allogeneic HSCT for adults with hemoglobinopathies. **Methods:** A comprehensive literature review was conducted using the PubMed database through January 2026, followed by a structured consensus-building process conducted by an expert panel. **Results:** Current evidence on indications for HSCT in adults with hemoglobinopathies was summarized, with emphasis on patient selection, conditioning regimens, graft-*versus*-host disease prophylaxis and management, and recommendations for long-term follow-up. **Conclusion:** Allogeneic HSCT from an HLA-identical sibling remains the standard of care for eligible adults with sickle cell disease. In addition, HSCT from matched unrelated and haploidentical donors has emerged as a promising therapeutic option, supported by encouraging outcomes in selected patients. In contrast, evidence regarding HSCT in adults with thalassemia remains limited, precluding definitive recommendations.

Keywords: Hemoglobinopathies; Hematopoietic Stem Cell Transplantation; Sickle Cell Disease. Thalassemia.

INTRODUCTION

Hemoglobinopathies are the most common monogenic diseases worldwide[1]. In Brazil, allogeneic hematopoietic stem cell transplantation (HSCT) has been approved within the public health system for thalassemia since 1999 and for sickle cell disease (SCD) since 2015 (Ministry of Health Ordinance No. 2,139 and Ordinance No. 1,321). Excellent outcomes were documented following HSCT for SCD from human leukocyte

antigen (HLA)-identical sibling donors, with overall survival (OS) ranging from 91 to 100% and event-free survival (EFS) ranging from 73 to 100%[2]. Evidence regarding HSCT in adults with thalassemia remains limited, precluding definitive recommendations.

This article aimed to provide an updated version of the Brazilian consensus published by the Brazilian Society of Cellular Therapy and Bone Marrow Transplantation, highlighting recent advances in the treatment of adult patients with SCD undergoing allogeneic HSCT.

METHODS

A comprehensive literature review was conducted using the PubMed database through January 2026, followed by a structured consensus-building process conducted by an expert panel.

RESULTS

SICKLE CELL DISEASE

Indications for allogeneic HSCT are summarized in Table 1. The decision should be individualized, as disease progression is highly variable[3]. Age is not a contraindication to HSCT. Eligible patients should undergo comprehensive organ function evaluation and assessment for SCD-related complications (Table 2)[4,5]. Vascular abnormalities consistent with a moyamoya syndrome pattern are not a contraindication, and pre-transplant surgical correction is not recommended.

Table 1. Indications for allogeneic hematopoietic stem cell transplantation in sickle cell disease.

Complication	Besides, consider
Overt stroke, silent stroke or cerebrovascular disease related to sickle cell disease	Administration of regular red blood cell transfusion therapy, defined as receiving eight or more transfusions per year for at least one year to prevent vaso-occlusive complications (*i.e.*, pain, stroke, and acute chest syndrome);
Two or more severe vaso-occlusive crises, including acute chest syndrome, in the last year	
More than one episode of priapism	
Presence of more than one erythrocyte alloantibody in patients receiving a hypertransfusion regimen	An echocardiographic finding of a tricuspid regurgitant jet velocity > 2.7 m/s.
Osteonecrosis in more than one joint	

Contraindication: severe comorbidities that increase transplant related mortality. Source: Elaborated by the authors.

Table 2. Pre-hematopoietic stem cell transplantation evaluation.

Organ/system	Evaluation
Lung	Pulmonary function test; lung tomography (lung parenchyma complications, thromboembolic events).
Heart	Echocardiogram with tricuspid valve evaluation; electrocardiogram; cardiologist evaluation if cardiac complications.
Central nervous system	Brain magnetic resonance angiography; neurological and psychiatric evaluation if necessary (pain management and neurological complications).
Liver	Liver T2-weighted magnetic resonance imaging (according to the number of transfusions and serum ferritin); abdominal ultrasonography (cholelithiasis and spleen evaluation, consider cholecystectomy before hematopoietic stem cell transplantation if cholelithiasis).
Kidney	Glomerular filtration rate; urinalysis; microalbuminuria-creatinine ratio.
Hematological system and transfusion complications	Hemolysis markers; reticulocyte count; fetal hemoglobin levels; anti-human leukocyte antigen antibody testing (donor-specific antibodies, in the setting of mismatched donors); extended erythrocyte phenotyping or genotyping; assessment of erythrocyte alloimmunization (historical and current); prior transfusion history; evaluation of iron overload; maintenance of sickle hemoglobin levels < 30%.
Multidisciplinary evaluation	Social work; psychology; blood transfusion; nutrition (identify and treat malnutrition); ophthalmology; dentist (perform dental procedures before hematopoietic stem cell transplantation); endocrinology; gynecology (consider fertility preservation before hematopoietic stem cell transplantation); pain evaluation and neurology (management of chronic pain); psychiatry (if pre-existing psychiatric disease, anxiety or depression).

Source: Elaborated by the authors.

Human leukocyte antigen identical sibling donors

The currently recommended conditioning regimen is myeloablative, based on the use of busulfan (Bu) (total dose 14–16 mg/kg) and cyclophosphamide (Cy) (total dose 200 mg/kg), combined with antithymocyte globulin (ATG)[6–9]. Despite excellent outcomes, this regimen is associated with increased morbidity and mortality and may be contraindicated in adults with significant comorbidities or organ dysfunction[10]. A reduced toxicity myeloablative regimen using fludarabine (Flu), Bu and ATG has shown promising results[11]. A nonmyeloablative, chemotherapy-free conditioning regimen, combining alemtuzumab and total body irradiation (TBI) has been successfully reproduced by other centers and represents a suitable option for adults and patients with established organ damage[12–14]. Recommended stem cell sources are bone marrow or cord blood, and graft-*versus*-host disease (GVHD) prophylaxis consists of cyclosporine plus methotrexate. In umbilical cord blood transplantation, methotrexate should be replaced with another immunosuppression agent.

Alternative donors

Haploidentical HSCT with post-transplant Cy in SCD was initially associated with high graft failure rates. Subsequent improvements in conditioning regimens—including the incorporation of pre-HSCT immunosuppression, escalation of TBI dose from 200 to 400 cGy, and the addition of thiotepa—have significantly reduced rejection rates[15–18]. Haploidentical HSCT is considered a viable option for patients with established indications.

Transfusion support

Alloimmunization occurs in approximately 20–50% of SCD patients[19]. The number of prior transfusions, history of transfusion reactions, presence of acquired anti-erythrocyte antibodies (AEA), and red blood cell (RBC) phenotyping are essential for optimal HSCT planning. Pre-HSCT evaluation should include, in addition to ABO and Rh typing, AEA screening, antibody titration in cases of ABO major or bidirectional incompatibility, a direct antiglobulin test, and extended RBC phenotyping, including C (RH2), E (RH3), c (RH4), e (RH5), K (KEL1), k (KEL2), Jk^a (JK1), Jk^b (JK2), Fy^a (FY1), Fy^b (FY2), S (MNS3), and s (MNS4). Genotyping is recommended to clarify complex cases and to identify RHCE variants[5,20,21].

Chimerism evaluation

Chimerism assessment is recommended after engraftment (day +30) and should be repeated on days +100, +120, +150, +180, and +365, as well as immediately before, during, and after immunosuppression withdrawal. In SCD, Bernaudin et al.[9] showed that 44% of patients undergoing HLA-identical sibling HSCT maintained mixed chimerism one year after transplantation without graft failure or recurrence of disease manifestations. Chimerism analysis should preferably be performed in specific cell populations (erythroid, myeloid, and T cells) rather than in whole peripheral blood[20]. Strategies to manage declining chimerism are not well established, although most authors recommend intensifying immunosuppression. Progressive reduction of donor chimerism in patients who underwent HLA-identical sibling HSCT is a possible indication for donor lymphocyte infusion (DLI), especially when chimerism falls below 30%, considering the associated risk of GVHD[22]. This approach cannot be extrapolated to haploidentical HSCT due to limited data on mixed chimerism. A Brazilian group reported, at a scientific meeting, improved donor chimerism in three out of four patients who underwent haploidentical HSCT for SCD and received DLI[23].

Iron overload

Whenever possible, optimal management of iron overload should be implemented during the pre-HSCT period[24–26]. Pre- and post-HSCT evaluation and management of iron overload are summarized in Table 3.

Table 3. Recommendations for the evaluation and management of iron overload.

	Evaluation	Monitoring
Before hematopoietic stem cell transplantation	Ferritin; transferrin saturation; magnetic resonance imaging for liver iron concentration (LIC) and T2-weighted; elastography, if available	Hematologic, renal, and hepatic function
Post-hematopoietic stem cell transplantation	Ferritin; transferrin saturation; magnetic resonance imaging (T2-weighted and LIC) only if clinically indicated or in patients with pre-HSCT abnormalities	Hematologic, renal, and hepatic function. In patients without transplant-related complications and not receiving transfusion support, consider evaluation every three months. More frequent evaluations should be guided by clinical and laboratory parameters
While receiving iron chelation therapy	Ferritin; transferrin saturation; magnetic resonance imaging (T2-weighted and LIC)	Hematologic, renal, and hepatic function every three months. More frequent evaluations should be guided by clinical and laboratory parameters

Source: Elaborated by the authors.

Long-term follow-up

Late complications vary according to patient age, disease status at HSCT and transplant-related factors[27]. Specific assessments require careful attention (Table 4)[11,28,29]. Antiepileptic therapy and magnesium supplementation should be considered to prevent neurological complications during immunosuppression, and blood pressure control is recommended[30,31]. Immunosuppression is usually discontinued approximately one year after HSCT.

Table 4. Long-term follow-up after hematopoietic stem cell transplantation for hemoglobinopathies.

Evaluation and exams	D+30	D+100	D+120	D+150	D+180	9M	12M	18M	24M	Annual
Disease evaluation (complete blood count, hemolysis markers, sickle hemoglobin analysis)	X	X	X	X	X	X	X	X	X	X
Chimerism	X	X	X	X	X	X	X	X	X	X
General exams (hepatic and renal function, biochemistry exams, urine analysis for blood/protein, microalbuminuria)	X	X	X	X	X	X	X	X	X	X
Brain magnetic resonance imaging and magnetic resonance angiography and transcranial Doppler velocity (for sickle cell disease)*							X		X	
Cardiac and hepatic magnetic resonance imaging (if abnormalities in previous exams)**							X		X	
Thyroid function-thyroid-stimulating hormone/T4L					X		X		X	X
Ferritin and transferrin saturation**					X		X		X	
Echocardiogram***							X		X	X
Pulmonary function tests***		X			X	X	X	X	X	
Lipid/glucose profile					X		X		X	X
Bone health: - Vitamin D (25-OH) level; - Bone mineral density#							X		X	X
Vaccination (according to institutional protocol)		X			X		X		X	

Continue...

Continuation.

Evaluation and exams	D+30	D+100	D+120	D+150	D+180	9M	12M	18M	24M	Annual
Fertility evaluation: follicular-stimulating hormone, luteinizing hormone, estradiol, anti-mullerian hormone, testosterone and sperm analysis (for men)							X			
Immune reconstitution[§]: Absolute numbers of NK cells, CD4+ and CD8+ T cells, B cells, and T-regs**		X			X	X	X	X	X	X
Visual function: vision test, glaucoma screening, retinal vessel assessment							X		X	X
Dental evaluation							X		X	X
Screening for malignancy							X	X	X	X

*Repeat every five years, or earlier if clinically indicated due to the development of symptoms; **annually until normalization; ***annually thereafter for five years, and beyond five years if abnormalities are detected or persist; [#]at one year and subsequently if an intervention is planned; [§]include post-vaccination specific antibody titers (tetanus, diphtheria, pneumococcus). Source: Elaborated by the authors.

CONCLUSION

Hemoglobinopathies are the most common monogenic disorders worldwide, and allogeneic HSCT remains the only available and safe curative therapy. Allogeneic HSCT from an HLA-identical sibling remains the standard of care for eligible adults with SCD. In addition, HSCT from matched unrelated and haploidentical donors has emerged as a promising therapeutic option, supported by encouraging outcomes in selected patients. In contrast, evidence regarding HSCT in adults with thalassemia remains limited, precluding definitive recommendations.

CONFLICT OF INTEREST

Nothing to declare.

DATA AVAILABILITY STATEMENT

Data sharing is not applicable.

AUTHOR'S CONTRIBUTIONS

Substantive scientific and intellectual contributions to the study: Costa TCM, Seber A, Barros GMN, Feliciano JVP, Gouveia RV, Albino CD, Helman R, De Santis GC, Rocha V and Simões BP; **Conception and design:** Costa TCM and Simões BP; **Acquisition of data:** Costa TCM, Seber A, Barros GMN, Feliciano JVP, Gouveia RV, Albino CD, Helman R, De Santis GC, Rocha V and Simões BP; **Analysis and interpretation of data:** Costa TCM, Seber A, Barros GMN, Feliciano JVP, Gouveia RV, Albino CD, Helman R, De Santis GC, Rocha V and Simões BP; **Manuscript preparation:** Costa TCM, Seber A, Barros GMN, Feliciano JVP, Gouveia RV, Albino CD, Helman R, De Santis GC, Rocha V and Simões BP; **Manuscript writing:** Costa TCM and Simões BP; **Critical revision:** Costa TCM, Seber A, Barros GMN, Feliciano JVP, Gouveia RV, Albino CD, Helman R, De Santis GC, Rocha V and Simões BP; **Final approval of the version to be published:** Costa TCM.

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DECLARATION OF USE OF ARTIFICIAL INTELLIGENCE TOOLS

The authors declare that no artificial intelligence tools were used in the preparation, writing, data analysis, or review of this manuscript.

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