Table 9. Recommendations for allogeneic hematopoietic stem cell transplantation

General	• Assessment of iron overload (MRI liver/heart) before planning HSCT • Iron overload: chelation prior HSCT, consider phlebotomies post HSCT
Age	• In general, before the age of 10 years in chronically transfused patients • If possible, preferably at the pre-school age (~2–5 years) to minimize risk of toxicities • In individual patients, HSCT for transfusion dependence can be considered after the age of 10 years (low transfusion burden, optimal iron balance, adequate organ function) • In adults, HSCT is generally not advised solely for the avoidance of transfusion dependence[1]
Indications	**Listed in order of increasing urgency and clinical necessity:** • Chronic transfusions in patients not responding to steroids • Chronic transfusions in patient with non-manageable iron overload (significant toxicity or chelator failure) • Chronic transfusions in patient with alloimmunization to RBC • Severe immunodeficiency and/or multilineage cytopenia • MDS/AML
Donor choice	**Donors listed with most optimal first:** • MSD: after exclusion of DBA syndrome in potential donor (genetic testing, CBC, eADA) • MUD: 10/10 HLA match based on molecular testing • MMUD and MMFD[2]: only in the absence of alternative therapies (patients with MDS/AML) or in context of clinical trials
Conditioning regimen	• Myeloablative (busulfan or treosulfan) regimen combined with fludarabine • Consider addition of thiotepa • Avoid irradiation
Stem cell source	• Bone marrow (any donor) • Cord blood (healthy sibling donor) • Avoid unmanipulated mobilized peripheral blood stem cells
GVHD prophylaxis	• Standard GVHD prophylaxis i.e., calcineurin inhibitor plus MTX or MMF and serotherapy (also for MSD)

[1] to be considered on a case-by-case basis for transfusion-dependent young adults in good health, after weighing the risks and benefits.
[2] includes haplo-donors.

Abbreviations: HSCT, hematopoietic stem cell transplantation; MDS, myelodysplastic syndrome; AML, acute myeloid leukemia; MSD, HLA-matched sibling donor; MUD, HLA-matched unrelated donor; MMUD, HLA-mismatched unrelated donor; MMFD, HLA-mismatched family donor; CBC, complete blood counts; eADA, erythrocyte adenosine deaminase; MTX, methotrexate; MMF, mycophenolate mofetil.