Cook Children's

When to refer for stem cell transplantation

Hematopoietic cell transplantation is a potential life-saving option for some patients. Early referral is one of the critical factors in improved outcomes, allowing optimum treatment planning and identification of donors. The guidelines on the back highlight disease categories that include patients at risk for disease progression or patients who should be referred for consultation.

Human leukocyte antigen (HLA) typing

If an allogeneic transplant is a treatment option, HLA typing of the patient, the patient's siblings and potential family donors is performed. Our referral HLA lab performs high-resolution DNA typing, which is essential if an unrelated donor transplant may be indicated. You may contact a Stem Cell Transplant program coordinator to arrange for HLA typing.

Preliminary unrelated donor searches

If no family match is found and an unrelated donor transplant is a possible treatment option, we perform searches through the National Bone Marrow Donor Program, Bone Marrow Donors Worldwide and many cord blood registries around the world. The preliminary search is done at no cost to the patient and provides an indication of the chances of finding a donor.

Formal unrelated donor searches

If an unrelated donor transplant is the treatment choice, a formal search is started. Donors are screened to confirm a match. These searches can take from two weeks to many months, depending on the patient's unique HLA type. Donor searches for minority patients may be more difficult.

Cook Children's Stem Cell Transplant physician team

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To refer, you may contact a Cook Children's Stem Cell Transplant coordinator:

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To better serve our treating clinicians, we can assist you with:

- Non-emergent transfer requests
- Direct admissions
- Specialist consultations

Cook Children's is actively involved in clinical research and is an active member of the Children's Oncology Group (COG), the Center for International Blood and Marrow Transplant Research (CIBMTR), the National Marrow Donor Program (NMDP), the Pediatric Blood and Marrow Transplant Consortium (PBMTC) and the Clinical Trials Network (CTN). We are also accredited through the Foundation for Accreditation of Cellular Therapy (FACT).

For some patients, early transplant may be indicated. For other patients, transplant may be needed later or not at all. Appropriate planning and early donor identification is critical to optimal patient outcomes.

Disease	When to refer	Notes
Acute myeloid leukemia (AML)	At diagnosis Primary induction failure or at relapse Patients with FLT3-ITD Treatment related AML Patients with antecedent myelodysplastic syndrome (MDS)	• HLA typing of all AML patients **at diagnosis** • Transplant in complete remission (CR) 1 if matched family member except favorable risk: t(8;21), t(15;17), inv(16) • Transplant in CR 2 and beyond if relapse
Acute lymphoblastic (ALL), standard-risk or high-risk	At relapse	• HLA typing and offer transplant as a therapy option
ALL, very high-risk	At diagnosis	• HLA typing and consider transplant for induction failure, +MRD after induction, severe hypodiploidy, CR2 and beyond
Juvenile myelomonocytic leukemia (JMML)	At diagnosis	• HLA typing for all patient at diagnosis • Transplant is only known definitive therapy
Chronic myelogenous leukemia (CML)	At diagnosis	• HLA typing and consider transplant if inadequate hematologic or cytogenetic response after trial of tyrosine-kinase inhibitor (TKI), accelerated phase or blast crisis; intolerance of TKI
Myelodysplastic syndrome (MDS)	At diagnosis	• HLA typing and consider transplant for all patients, timing dependent on clinical course
High-risk neuroblastoma	At diagnosis	• Referral at diagnosis allows for planning stem cell collection • We participate in the New Approaches to Neuroblastoma Therapy (NANT) consortium and many non-transplant protocols including MIBG and/or antineoplastic agents may require stem cell rescue
Non-Hodgkin's lymphoma	Primary induction failure or at relapse	• Either autologous or allogeneic transplant may be considered • Early referral allows for planning stem cell collections and/or donor search
Hodgkin's lymphoma	Primary induction failure or at relapse	• Early referral allows for planning stem cell collection
Severe aplastic anemia	At diagnosis	• Transplant prior to immunosuppressive therapy if matched family member • Preliminary unrelated donor search if no family match
Genetic syndromes (e.g., Hurler syndrome, Adrenoleukodystrophy (ALD) and others)	At diagnosis	• Full evaluation required to determine suitability and appropriateness of transplant • Early transplant provides best patient outcome
Sickle Cell disease	Frequent pain crisis, stroke, abnormal transcranial doppler (TCD), acute chest syndrome	• Consider in all patients though evaluation required to determine suitability and appropriateness
Transfusion - dependent thalassemia	At diagnosis	• Preferably prior to age 5 or before heavy transfusion support/iron overload
Fanconi anemia	At diagnosis	• HLA typing and treatment planning
Diamond Blackfan anemia	If steroid dependent	• HLA typing and treatment planning
Histiocytic/hemophagocytic disorders	Familial hemophagocytic lymphohistiocytosis (FEL): at diagnosis; Langerhans cell histiocytosis (LCH): when refractory	• Full evaluation required to determine suitability and appropriateness of transplant • Early transplant provides best patient outcome
Wiskott Aldrich syndrome and other immunodeficiencies	At diagnosis	• HLA typing and treatment planning if transplant is an option
Severe combined immune deficiency (SCID)	At diagnosis	• Urgent transplant usually required

Other diseases to consider: bone marrow failure syndromes, other hemoglobinopathies, Glanzmann's thrombasthenia, relapsed high risk solid tumors and some CNS tumors