

PEDIATRIC HSCT UNPLUGGED:

BREAKING MYTHS, KNOWING INDICATIONS

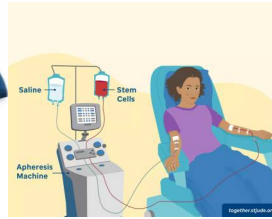
What are Hemopoietic Stem cells?

- ▶ Present in the bone marrow
- ▶ Give rise to red blood cells, white blood cells and platelets
- ▶ Blood stem cells lie in a spongy bed that provide nourishment - Seed and soil concept

Bone Marrow Harvesting



Apheresis



Stem cell donation is safe.
HLA match is key for donor selection.
Blood group mismatch is not a barrier.

What is Hematopoietic Stem Cell Transplantation – HSCT or BMT?

- ▶ HSCT is the reconstitution of the entire hematopoietic system by transfer of pluripotential stem cells

What are the types of transplantation ?

- ▶ Autologous – Stem cells come from the patient's own body
- ▶ Allogeneic – Stem cells come from a healthy donor

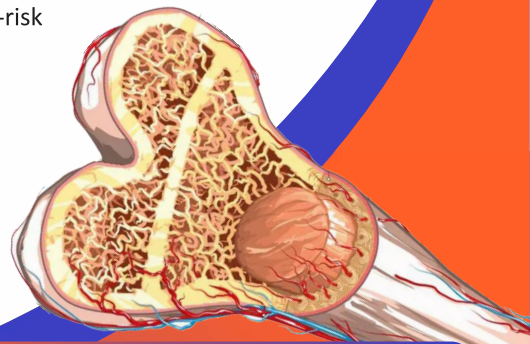
HSCT Indications in Malignant Diseases (Cancers)

Autologous HSCT

1. High Risk Neuroblastoma
2. High risk / Relapsed Germ Cell Tumors
3. Infant Medulloblastoma
4. Hodgkin lymphoma, for relapsed or refractory cases
5. Non-Hodgkin Lymphoma, for relapsed or refractory cases

Allogeneic HSCT

1. Acute Lymphoblastic Leukemia (ALL), - very high-risk & relapsed disease
2. Acute Myeloid Leukemia (AML), high-risk subtypes and relapse cases
3. Chronic Myeloid Leukemia (CML) in blast crisis
4. Juvenile Myelomonocytic Leukemia (JMML)
5. Myelodysplastic Syndrome (MDS)



What are the conditions for which we use allogeneic HSCT?

Severe Combined Immune Deficiency (SCID)	Wiskott-Aldrich Syndrome (WAS) Hyper IgM Syndrome	Chronic Granulomatous Disease (CGD) Leukocyte Adhesion defect (LAD)
X linked Agammaglobulinemia Hemophagocytic Lymphohistiocytosis (Primary HLH)	HSCT Indications in Inborn Errors of Immunity (IEI)	Severe Congenital Neutropenia
Monogenic very early onset Inflammatory Bowel Disease	Mendelian Susceptibility to Mycobacterial Diseases (MSMD)	

What are the conditions for which we use allogeneic HSCT?

Hemoglobinopathies Transfusion Dependent Thalassemia Sickle Cell Disease	Bone Marrow Failure Severe Aplastic Anemia HSCT Indications in Non Malignant Conditions	Disorders of platelet function: Glanzmann Thrombasthenia Inherited Metabolic and Genetic Disorders Hurler Syndrome MPS type I Adrenoleukodystrophy
IBMFS: Fanconi Anemia Diamond-Blackfan Anemia CAMT Transfusion Dependent Pure Red Cell Aplasia	Gaucher's Disease Osteopetrosis Maroteaux-Lamy Syndrome (MPS-VI)	

Myths and Facts About Bone Marrow / Stem Cell Transplantation in Children

Myth 1: BMT is the last resort and always indicated after the failure of previous treatments

Fact: BMT is a planned curative therapy for various conditions, including thalassemia, bone marrow failure syndromes, and some immune deficiencies. It's not always a last resort.

Myth 2: Siblings are always the best and only donors

Fact: While matched siblings are ideal, many children undergo successful transplants with Matched Unrelated Donors (MUD) and haploidentical donors (parents)

Myth 3: Children become permanently immunocompromised & must live in a sterile "bubble" forever after the BMT.

Fact: After the initial recovery period, most children regain normal immunity and will be off immunosuppression by 12-18 months

Myth 4: Stem cell donation is dangerous for the donor.

Fact: Peripheral Blood Stem Cell (PBSC) and bone marrow donation, both are very safe procedure for the donor. Serious complications for donors are extremely rare.



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