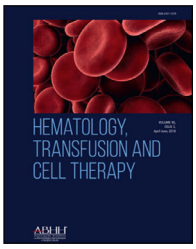
HEMATOLOGY, TRANSFUSION AND CELL THERAPY

www.htct.com.br



PEDIATRIC PRESENTATIONS

Sp01

TREATMENT OF SICKLE CELL ANEMIA

Dr. Salam Alkindi

Professor & Senior Consultant, department of hematology, college of medicine & health sciences, Sultan Qaboos University, Muscat, Oman

Sickle cell disease (SCD) is an inherited disorder prevalent in many areas of the world including Africa, Middle East and parts of India. It is characterized by repetitive episodes of vaso-occlusive (VOC) process, leading to recurrent painful episodes, hemolytic anemia and predisposition to infection. Although VOC is a leading manifestation of SCD, and seen in about 90% of all patients with SCD, however organ specific complications such as acute chest syndrome, stroke, splenic sequestration, and many skeletal complications are also seen. Better understanding of pathophysiology of the disease as well as worldwide interest in the disease has allowed more progress on treatment and prevention of these complications and development of more focused pharmacological therapies. Hemoglobin polymerization is a primary triggering event in the pathophysiology of the disease, resulting in vascular injury and leading to the process of sickling. This usually ignite an intense inflammatory process/ tissue ischemia and increased adhesions. This understanding of the pathophysiology has allowed scientist to develop drugs (three FDA approved within the last few years), that interfere with these processes such as Voxelotor & Hydroxyurea (interfere with polymerization and enhance HbF production), L-glutamine and Omega 3 (interfere with inflammatory process and oxidative stress) and Crizanluzimab and Tinzaparin (works by inhibiting adhesion molecules). Others studies looking at similar and other pathways are ongoing, including drugs that improve adenosine triphosphate (ATP) levels and reducing 2,3-diphosphoglycerate (2,3-DPG) levels. The availability of these therapeutic interventions, will allow patients and physicians the freedom to have patient specific therapeutic interventions including development of combinations protocols. SCD is very complex and this meant that drug with multi-faceted action such as Hydroxyurea will remain with us for some time. Further progress also made in the area of bone marrow transplant (including alternative donor pool) and gene therapy /gene editing, with recently published data is very encouraging. Although the prognosis of patients with SCD has improved, due to introduction of vaccination, use of antibiotics prophylaxis and blood transfusions, however still patients are dying prematurely and further work is needed on understanding disease and its manifestation.

https://doi.org/10.1016/j.htct.2022.09.1202

Sp02

EARLY T-CELL PRECURSOR ACUTE LYMPHOBLASTIC LEUKEMIA IN CHILDHOOD

Hale Ören

Dokuz Eylül University Faculty of Medicine, Department of Pediatric Hematology, İzmir, Türkiye

Early T-cell precursor (ETP) ALL accounts for 10% to 15% of T-ALL, which arises from an early T-cell lineage clone with aberrant expression of myeloid and/or early progenitor cell markers (1,2). ETPs are a subset of thymocytes representing recent immigrants from the bone marrow to the thymus, they retain multilineage differentiation potential, suggesting their direct derivation from hematopoietic stem cells (3). ETP-ALL, which was first reported by Coustan-Smith in 2009, largely overlaps with the pro-T subtype of the EGIL classification; its special diagnostic criteria in immunophenotypic screening are the absence of CD1a and CD8 expression, the absence or weak expression of CD5, and the presence of strong positive for at least one of CD34, CD117, HLADR, CD13, CD33, CD11b, and CD65 (2,4-6). In case of strong positivity of CD5, at least two of the latter must be strong positive (6). There is also novel evidence that the myeloid marker CD371 may be positive in ETP (6). The genetic features of ETP-ALL are similar to those of hematopoietic stem cells and myeloid progenitor cells. The genomic mutations of ETP-ALL are enriched in hematopoietic transcriptional regulators (such as BCL11B, ETV6, RUNX1, biallelic WT1, and GATA3), epigenetic factors