Section: Pediatrics

Original Research Article

A STUDY OF CLINICAL PROFILE AND EARLY OUTCOME OF PEDIATRIC BONE MARROW TRANSPLANT

Disha Tiwari[1], Tanmesh Sahu[1], Prashant Pandey[2]

[1]*Assistant Professor, Department of Pediatrics, MGM Medical College, Panvel, Maharashtra, India.*
[2]*Department of Orthopaedics, DY Patil School of Medicine, Nerul, Maharashtra, India.*

Received *: 07/03/2026*
Received in revised form *: 26/04/2026*
Accepted *: 12/05/2026*

Corresponding Author:
Dr. Disha Tiwari,
Assistant Professor, Department of Pediatrics, MGM Medical College, Panvel, Maharashtra, India.
Email: disha1994@live.com

DOI: 10.70034/ijmedph.2026.2.614

Source of Support: Nil,
Conflict of Interest: None declared

Int J Med Pub Health
2026; 16 (2); 3731-3737

ABSTRACT

Background: Allogeneic hematopoietic stem cell transplantation (HSCT) has become the only known curative treatment of a variety of hematologic disorders in children, particularly, transfusion-dependent thalassemia, sickle cell anemia, and severe aplastic anemia. The conditioning regimens based on busulfan, fludarabine, cyclophosphamide and serotherapy (ATG) have developed to enhance the engraftment and reduce the level of toxicity, but in the low- and middle-income environment, the initial infectious and non-infectious complications are still incidental. Outcomes of 30 days after pediatric HSCT were measured in a tertiary pediatric HSCT center.

Materials and Methods: Children under 18 years subjected to HSCT after agreement with ethics and parents were enrolled in a prospective retrospective cohort of observational study. Baseline clinical status, characteristics of the donors, conditioning, dose of the stem-cells, and peri-transplant occurrences were documented. The early day 30 outcome included whole-blood donor chimerism; infection loads during the conditioning and the first 30 days after the HSCT, engraftment pattern, transfusion needs, and early complications.

Results: The cohort consisted of thalassemia major (70.2%), sickle cell anemia (13.5%), aplastic anemia (10.8), myelodysplasia (2.7), and CML (2.7). As of day 30, the percentages of donor chimerism were more than 95% in 24/37 (64.9%); partially (5-95) in 10/37 (27.0%); less than half in 1/37 (2.7%); and 4/37 (10.8). Underweight (weight-for-age) was a highly significant predictor of poor early outcome (X 2=22.020; p=0.037). By days <14, 14 21, and >28, neutrophil engraftment was performed achieved within 30 days of transplantation as compared to platelet engraftment. Culture- negative sepsis (50% of the 36 total post-HSCT febrile episodes) and fungal sepsis (13.8%) were the most common sepsis types; CMV reactivation was 8.3%. Non-infectious complications as mucositis (43.2%) and hypertension (35.1%) and less frequent veno-occlusive disease (13.5) and infrequent GVHD (2.7) were observed. Mainly central venous lines were electively removed beyond day 28.

Conclusion: This Indian Pediatric HSCT programme has early results which are satisfactory as by day 30, two-thirds have attained complete donor chimerism. Nutritional status was found to be a risk correlate that could be modified, whereas the early morbidity was predominant by infections especially by culture-negative sepsis and non infectious complication of mucositis. Optimization and infection prevention of pre-HSCT can be strengthened and further supplement a better outcome in early results.

Keywords: Pediatric HSCT; thalassemia; conditioning regimen; chimerism; engraftment pattern; infections; mucositis; India.

INTRODUCTION

Bone Marrow Transplantation (BMT), presently termed Hematopoietic Stem Cell Transplantation (HSCT), is a potentially curative therapeutic modality for a wide spectrum of malignant and non-malignant hematological disorders, immunodeficiency syndromes, inherited metabolic diseases, and genetic disorders in children. Over the last few decades, HSCT has emerged as one of the most significant advances in pediatric medicine, substantially improving survival and quality of life in children suffering from otherwise fatal illnesses.[1-3]

HSCT involves the intravenous infusion of hematopoietic stem cells with the objective of re-establishing normal hematopoiesis and immune function following myeloablative or reduced-intensity conditioning therapy. Stem cells may be obtained from bone marrow, peripheral blood, or umbilical cord blood sources. Advances in donor selection, human leukocyte antigen (HLA) matching, graft engineering, supportive care, antimicrobial prophylaxis, and immunosuppressive therapy have significantly enhanced transplant success rates worldwide.[2,4]

In the pediatric age group, HSCT has become an established treatment option for acute lymphoblastic leukemia, acute myeloid leukemia, severe aplastic anemia, thalassemia major, sickle cell disease, primary immunodeficiency disorders, hemophagocyticlymphohistiocytosis, inherited bone marrow failure syndromes, and several metabolic disorders. The expanding indications for transplantation have resulted from continuous improvements in transplant techniques and supportive care strategies.[2,5]

Despite remarkable therapeutic advances, HSCT remains associated with substantial morbidity and mortality. The transplantation process exposes children to intensive conditioning regimens, prolonged immunosuppression, and multiple infectious and non-infectious complications. Early transplant-related complications include febrile neutropenia, bacterial and fungal infections, mucositis, graft failure, hepatic dysfunction, veno-occlusive disease, engraftment syndrome, and acute graft-versus-host disease (GVHD). These complications significantly influence transplant-related mortality and overall outcomes.[4,6,7]

Graft-versus-host disease remains one of the most serious complications following allogeneic HSCT. It occurs when immunocompetent donor lymphocytes recognize recipient tissues as foreign and initiate an inflammatory immune response. Acute GVHD commonly affects the skin, liver, and gastrointestinal tract and contributes significantly to post-transplant morbidity and mortality. Improved donor matching and newer prophylactic strategies have reduced, but not eliminated, the burden of GVHD in pediatric transplant recipients.[2,4,8]

The early post-transplant period, generally defined as the first 100 days following HSCT, represents a particularly vulnerable phase characterized by profound immunosuppression, delayed immune reconstitution, and increased susceptibility to infections and organ toxicities. Assessment of early outcomes during this period, including engraftment kinetics, graft failure, transplant-related complications, intensive care requirements, duration of hospitalization, and short-term survival, provides critical information regarding the effectiveness and safety of transplant programs.[1,6]

Engraftment is considered one of the earliest indicators of transplant success. Successful neutrophil and platelet engraftment reflects restoration of hematopoietic function and is associated with improved clinical outcomes. Delayed engraftment or graft failure may result in prolonged hospitalization, recurrent infections, increased transfusion requirements, and higher mortality. Recent pediatric studies have demonstrated favorable engraftment rates with advances in donor selection and transplant protocols.[1]

In developing countries such as India, the burden of diseases requiring HSCT remains considerable. Hemoglobinopathies such as thalassemia major, severe aplastic anemia, and hematological malignancies constitute major indications for pediatric transplantation. Although several tertiary care centers have established successful transplant programs, published data regarding the clinical profile and early outcomes of pediatric HSCT recipients remain relatively limited. Differences in disease spectrum, socioeconomic conditions, nutritional status, infectious disease burden, donor availability, and healthcare infrastructure may significantly influence transplant outcomes in the Indian setting.[1,2]

Systematic evaluation of the clinical characteristics, indications, complications, and early outcomes of pediatric HSCT recipients is essential for identifying prognostic factors, improving supportive care protocols, reducing transplant-related morbidity, and enhancing survival. Furthermore, institution-specific outcome data are necessary for benchmarking transplant performance and guiding future clinical decision-making.[1]

Despite the growing number of pediatric HSCT procedures being performed in India, data regarding the clinical profile, transplant-related complications, engraftment characteristics, and early post-transplant outcomes remain limited, particularly from individual tertiary care centers. Early identification of factors influencing transplant success is essential for optimizing patient selection, refining transplant protocols, and improving supportive care practices. A comprehensive evaluation of demographic characteristics, underlying diagnoses, transplant-related variables, infectious complications, engraftment patterns, transfusion requirements, and early morbidity may

provide valuable insights into determinants of transplant outcomes in children.

Therefore, the present study was undertaken to evaluate the clinical profile and early outcomes of pediatric patients undergoing hematopoietic stem cell transplantation at a tertiary care teaching hospital. Early outcome was assessed primarily by donor chimerism status on Day 30 following transplantation. Secondary outcome measures included engraftment kinetics, infection burden during conditioning and the early post-transplant period, transfusion requirements, and non-infectious complications. In addition, the study aimed to examine the association of factors such as anthropometric status, organomegaly, ABO incompatibility, stem-cell dose, and conditioning regimen with early transplant outcomes.

MATERIALS AND METHODS

Study design and setting: It was a prospective-retrospective observational study that was done at tertiary care centre in navimumbai. The research articles were between August 2024 and August 2025.

Participants: Children younger than 18 years with approved indications (e.g., transfusion-dependent-thalassemia, sickle cell anemia, aplastic anemia, or selected malignant disorders). The exclusion criteria included the absence of consent or absence of complete necessary outcome data.

Ethics: The Institutional Ethics Committee approved the protocol. Written informed consent was obtained from parents/guardians; assent was obtained where appropriate.

Pre-HSCT evaluation: On the initial inquiry, we received full history (childhood illnesses, immunizations, drugs allergic reactions, transfuses, disease onset and diagnostics, previous chemo/radiotherapy, treatment complications and relapses, family pedigree, and logistics). A physical examination was conducted. The status of disease and organ measurements were recorded; anthropometry (z weight-for-age and height-for-age) and organomegaly (liver /spleen) were banded using SDs. The HLA typing was performed on recipient and potential donors, matched sibling donor was given the first priority in the absence of which haploidentical and mismatched related donors were explored. Pre-transplant serology (CMV, VZV, HSV, toxoplasma, HBsAg, HIV, HCV) was done.

Conditioning and graft: The patients were exposed to conditioning using disease-appropriate, after receiving the central venous catheter (CVC). Regimens consisted of busulfan-based myeloablation using cyclophosphamide and ATG with or without fludarabine (cumulative 160 to 200 mg/m 2), and total body irradiation in malignant indications. The bone marrow or peripheral blood provided the stem cells which were introduced to the CVC with constant monitoring.

Peri-transplant care: Prophylaxis involved antibacterial, antifungal, antiviral coverage, and supportive treatment. Febrile episodes were considered to be 38.2 degrees and above. Infections were categorised as: culture-proven bacterial (blood/urine/sputum/pus/catheter) culture-negative sepsis, invasive fungal disease (serum galactomannan, CT chest/sinus and /or positive culture) and viral reactivation (CMV/EBV/adenovirus by PCR).

Outcomes: The major result was 30-day whole blood chimerism (complete >95%, mixed 5-95%, low <5%), infection burden during conditioning and the first 30 days following HSCT, neutrophil engraftment (Absolute neutrophil count> 500) during at least 3 consecutive days; platelet engraftment (platelet count> 20,000) for 7 days, non-infectious complications (mucositis, hypertension, VOD, GVHD, PRES), transfusion needs, CVC type and time of removal, and early mortality.

Statistics: Data were tabulated in Excel 2013 and analyzed with SPSS v2.0. Continuous variables were summarized as mean ± SD; categorical variables as counts and percentages. Associations with day-30 outcome were tested by χ^2 (df per comparison); $p<0.05$ (two-sided) denoted statistical significance.

RESULTS

Cohort and baseline characteristics: We enrolled 37 pediatric HSCT recipients: β-thalassemia major (n=26, 70.2%), sickle cell anemia (n=5, 13.5%), aplastic anemia (n=4, 10.8%), myelodysplastic syndrome (n=1, 2.7%), and CML (n=1, 2.7%). Males predominated across age strata. Donors spanned all pediatric age groups.

Anthropometry reflected prevalent undernutrition: weight-for-age between −1 and −2 SD in 40.5% and between −2 and −3 SD in 16.2%; height-for-age between 0 and −1 SD in 43.2% and −2 to −3 SD in 21.6%. Among hemoglobinopathy patients (n=31), hepatosplenomegaly was common: liver size >+2 SD in 22.5% and spleen >+2 SD in 24.3%.

Most recipients (83.7%) received a tunneled CVC (central venous line) and 16.2% a PICC. Stem-cell doses were <5, 5–<10, and >10 million/kg in 48.6%, 24.3%, and 27.0%, respectively. For conditioning was ATG/Bu/Cy (12.9%), ATG/Bu/Cy/Flu-160 (45.2%), and ATG/Bu/Cy/Flu-200 (35.1%).

Early outcomes and engraftment: By day 30, complete donor chimerism (>95%) occurred in 24/37 (64.9%); mixed chimerism (5–95%) in 10/37 (27.0%); <5% in 1/37 (2.7%); and death in 4/37 (10.8%). Disease-specific distributions were broadly similar (non-significant across indications). Recipient weight-for-age significantly associated with early outcome (χ^2=22.020, df=12, p=0.037): worse Z-scores correlated with lower rates of complete chimerism and higher early mortality.

Height-for-age, organomegaly, ABO incompatibility, stem-cell dose, and conditioning regimen showed no statistically significant associations with day-30 outcome in this sample.

Neutrophil engraftment occurred by <14 days in 9.0%, 14–<21 days in 51.5%, 21–<28 days in 30.3%, and >28 days in 9.0% while Platelet recovery lagged (3.0%, 27.2%, 27.2%, and 42.4% in the same intervals).

Infections and complications: During conditioning, 86.5% had 1–3 febrile episodes (1.59 episodes/recipient overall), commonly related to central line placement or ATG infusion. In the first 30 days post-HSCT, 36 febrile episodes occurred (0.97/recipient): culture-negative sepsis (50%), culture-proven bacterial sepsis (11.1%), CMV reactivation (8.3%), fungal sepsis (13.8%), and other causes (16.6%). Non-infectious complications included mucositis (43.2%), hypertension (35.1%), VOD (13.5%), and rare GVHD (2.7%) and PRES (2.7%). CVCs were most often removed electively after day 28; earlier removals were usually for emergency indications including uncontrolled sepsis.

Table 1: Disease distribution of pediatric HSCT recipients (N=37)

Disease	Number of recipients, n (%)
Thalassemia Major	26 (70.2)
Sickle Cell Anemia	5 (13.5)
Aplastic Anemia	4 (10.8)
Myelodysplastic Syndrome	1 (2.7)
Chronic Myeloid Leukemia	1 (2.7)

The cohort is representative of a hemoglobinopathy-dominant Indian pediatric program of the HSCT, transfusion-dependent thalassemia (~70% of procedures) and sickle cell disease (another 14%). Non malignant marrow failure (aplastic anemia) had a contribution of about 11% but malignant indications are not very common during this period.

Table 2: Weight-for-age vs day-30 outcome

Weight-for-age Z-score band	>95% donor chimerism (n)	5–95% donor chimerism (n)	<5% donor chimerism (n)	Death ≤30 days (n)	p-value (Pearson χ^2, df=12)
0 to +1 SD (n=3)	2	0	0	0	0.0373
0 to −1 SD (n=12)	11	0	0	1	0.0373
−1 to −2 SD (n=15)	9	4	0	2	0.0373
−2 to −3 SD (n=6)	2	3	1	0	0.0373
< −3 SD (n=1)	0	0	0	1	0.0373

Early outcomes were related to nutritional status. The mucosal integrity and innate immunity may be affected by malnutrition, making one more susceptible to infections, and dysfunctional in the context of hematopoietic recovery. The results support the need to optimize pre-HSCT nutrition based on dietetic examination, transfusion with micronutrients, and, if necessary, enteral nutrition to increase engraftment biology and post-transplant outcome.

Table 3. engraftment pattern among survivors to day 30 (n=33)

Post-HSCT day	Neutrophil engraftment	Platelet engraftment
<14	3 (9.0%)	1 (3.0%)
14–<21	17 (51.5%)	9 (27.2%)
21–<28	10 (30.3%)	9 (27.2%)
≥28	3 (9.0%)	14 (42.4%)

In the majority of recipients, myeloid recovery occurred as expected. Delays in platelet recovery - 30 percent at day 21 and approximately 70 percent at day 28 or later - in line with recommended trends of late megakaryopoiesis following myeloablative conditioning.

Table 4: Post-transplant febrile episodes within 30 days (N=36 episodes)

Etiology	Episodes n (%)
Culture-negative sepsis	18 (50.0)
Culture-proven bacterial sepsis	4 (11.1)
CMV reactivation	3 (8.3)
Fungal sepsis	5 (13.8)
Other causes	6 (16.6)

Culture- negative sepsis dominated early febrile morbidity which is seen in neutropenia that received previous antibiotics. Fungal disease (13.8), CMV reactivation (8.3) was also similar to pediatric HSCT literature. Strict diagnostic stewardship (in real time imaging, biomarkers) and pre-emptive antiviral surveillance contributed to this.

The stacked bar represents the relative share of culture-negative sepsis, culture-proven bacterial sepsis, CMV reactivation and invasive fungal

disease with early febrile events. The prevalence of culture-negative highlights the problems of microbiologic yield in conditions of neutropenia.

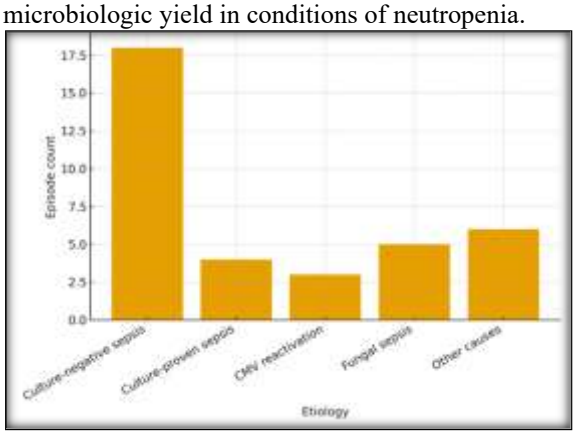

Figure 1: distribution of infectious etiologies among post-hsct febrile episodes

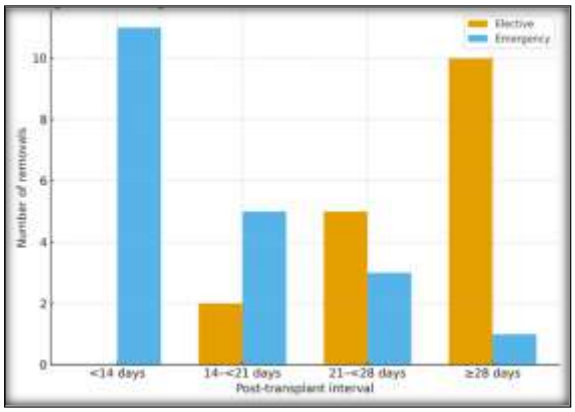

Figure 2: timing and indication for central venous catheter removal

Bar graphs of elective vs. emergency CVC removal in four post-HSCT periods. Early removal (less than 21 days) were mostly emergency considering complications of catheters or uncontrolled infection. Most of the removals subsequent to day 28, on the contrary, were elective, which corresponds to hematologic stability. Strict catheter-care bundles, early ultrasonography to evaluate thrombus and timely line salvage was done.

DISCUSSION

The present study included 37 pediatric HSCT recipients, with β-thalassemia major constituting the largest subgroup (70.2%), followed by sickle cell anemia (13.5%), aplastic anemia (10.8%), myelodysplastic syndrome (2.7%), and chronic myeloid leukemia (2.7%). Similar findings have been reported by Angelucci et al., where β-thalassemia major and sickle cell disease represented the most common non-malignant indications for pediatric HSCT.[9] Likewise, De Avila et al. demonstrated that hemoglobinopathies accounted for a substantial proportion of pediatric HSCT procedures in the United States.[10] Xiao et al. also reported a predominance of children with transfusion-dependent β-thalassemia undergoing HSCT, highlighting the continuing role of transplantation as a curative modality for hemoglobinopathies.[11] The male predominance observed in our cohort is consistent with several pediatric HSCT series, although sex distribution has generally not been shown to significantly influence transplant outcomes.

he present study demonstrated a high prevalence of undernutrition among pediatric HSCT recipients, with 40.5% of children having weight-for-age between −1 and −2 SD and 16.2% between −2 and −3 SD. Similarly, height-for-age deficits were common, with 21.6% exhibiting moderate stunting. Comparable findings have been reported by Moiz et al., who observed significant growth retardation and poor anthropometric indices among transfusion-dependent β-thalassemia patients.[12]Ara et al. reported that 44.7% of children were underweight and 40.3% had stunted growth, while Mirhosseini et al. documented a high prevalence of malnutrition and low BMI among pediatric thalassemia patients.[13,14] Furthermore, hepatosplenomegaly observed in our hemoglobinopathy cohort is consistent with previous reports describing organomegaly as a common consequence of chronic hemolysis, ineffective erythropoiesis, and iron overload in transfusion-dependent thalassemia.[15]

Several published pediatric HSCT studies have reported findings comparable to your observations regarding central venous access, stem-cell dose distribution, and busulfan-based conditioning regimens, particularly in children with transfusion-dependent β-thalassemia major.[16]

The majority of children in the present study received transplantation through tunneled central venous catheters (83.7%), while a smaller proportion underwent transplantation via PICC lines (16.2%). Similar observations have been reported by Martynov et al., who described tunneled central venous catheters as the preferred vascular access device in pediatric HSCT owing to the requirement for prolonged chemotherapy, stem-cell infusion, transfusion support, and intensive monitoring. Regarding graft characteristics, nearly half of the recipients received stem-cell doses below 5 million/kg, whereas the remaining patients received higher cell doses. Comparable variability in infused stem-cell dose has been reported across pediatric HSCT series involving hemoglobinopathies. Conditioning in our cohort was predominantly based on ATG, busulfan, cyclophosphamide, and fludarabine combinations. Similar Bu/Cy/Flu/ATG-based regimens have been widely reported by Seth V., who demonstrated excellent engraftment and survival outcomes among children with transfusion-dependent β-thalassemia undergoing allogeneic HSCT.

The present study demonstrated a high frequency of febrile episodes during conditioning and the early post-transplant period, with the majority of recipients experiencing at least one febrile event.

Similar findings have been reported by Zajac-Spychala O et al., who identified fever as one of the most common complications during conditioning and neutropenia following pediatricHSCT.[18] Culture-negative sepsis constituted the predominant cause of fever in our cohort, followed by bacterial sepsis, fungal infections, and CMV reactivation. Comparable patterns have been documented in pediatric transplant studies, where culture-negative febrile episodes frequently outnumber microbiologically confirmed infections. Non-infectious complications were dominated by mucositis, hypertension, and veno-occlusive disease. Agholme MB et al. reported mucositis as the most frequent toxicity associated with busulfan-cyclophosphamide-based conditioning,[19] while Bognàr T et al. observed VOD rates ranging from 10% to 20% in pediatric recipients receiving myeloablative regimens. The incidence of hypertension in the present study is also comparable to that reported by Kwon DH et al. Acute GVHD and PRES were uncommon, consistent with observations from pediatric non-malignant disease transplant cohorts receiving ATG-based prophylaxis.[20] Regarding vascular access, most central venous catheters were removed electively after hematological recovery, whereas early catheter removal was generally necessitated by severe infectious complications, findings similar to those reported by Martynov et al. in pediatric HSCT recipients.[16]

Limitations: First, the study was conducted at a single tertiary care center with a relatively small sample size of 37 pediatric HSCT recipients, which may limit the generalizability of the results to other institutions and populations. Second, the heterogeneous nature of the study population, comprising different underlying diseases such as β-thalassemia major, sickle cell anemia, aplastic anemia, myelodysplastic syndrome, and chronic myeloid leukemia, may have influenced the observed clinical outcomes and complication profiles. Third, the retrospective observational design relied on available medical records, making the study susceptible to incomplete documentation and information bias. Long-term follow-up data regarding growth, endocrine dysfunction, quality of life, chronic graft-versus-host disease, and late transplant-related complications were not available for all patients. Furthermore, detailed assessment of factors influencing transplant outcomes, including donor characteristics, HLA matching, pharmacokinetic monitoring of conditioning agents, and socioeconomic determinants, was beyond the scope of the study. The absence of a comparison group also limits the ability to establish causal relationships between clinical variables and transplantation outcomes.

Implications and future directions.

Despite these limitations, the study provides valuable insights into the demographic characteristics, nutritional status, transplant procedures, complications, and early outcomes of pediatric hematopoietic stem cell transplantation recipients in a resource-constrained tertiary care setting. The findings emphasize the importance of early referral and transplantation in children with transfusion-dependent hemoglobinopathies before the development of advanced organ damage and severe nutritional deficits. The high frequency of infectious complications and mucositis highlights the need for vigilant infection surveillance, optimized supportive care, and strict catheter management protocols during the peri-transplant period. The study also underscores the significance of pre-transplant nutritional assessment and intervention as an integral component of comprehensive transplant care. The favorable engraftment and survival outcomes observed with busulfan-based conditioning regimens support their continued use in appropriately selected pediatric patients. Furthermore, the results contribute to the growing body of Indian data on pediatric HSCT and may assist clinicians, transplant teams, and policymakers in developing evidence-based strategies to improve transplant outcomes. Larger multicentric prospective studies with long-term follow-up are warranted to validate these findings and further optimize pediatric HSCT practices.

CONCLUSION

Two-thirds of this pediatric HSCT cohort in a tertiary care teaching hospital has reached complete donor chimerism by day 30 with acceptable early mortality and complication rates. The pre-HSCT nutritional optimization is essential, as the malnutrition was associated with poor early outcomes. Culture-negative febrile episodes, mucositis and transfusion dependence as well as manageable rates of CMV reactivation fungal disease as well as VOD contributed to early morbidity. Additional interventions that may enhance early recovery consist of attention to busulfan exposure control, prevention of infections and catheter-care bundles. Extending follow up to one year and lineage specific chimerism and GVHD/ relapse surveillance will shed light on long term efficacy and safety.

REFERENCES

1. Kanakkande Kandy ZA, Nivargi S, Chaudhary B, Ramzan M, Abdullah F, Aboobacker F, Khalil C, Porta F, Faulkner L, Alshamsi H. Outcomes of Hematopoietic Stem Cell Transplantation (HSCT) From Matched Related Donors in Pediatric Patients: Insights From a Single Center in the United Arab Emirates. Cureus. 2025 Aug 2;17(8):e89239. doi: 10.7759/cureus.89239. PMID: 40904984; PMCID: PMC12402674.
2. Guilcher G, Rivard L, Huang J et al.Immune function in childhood cancer survivors: a Children's Oncology Group reviewThe Lancet Child & Adolescent Health, 2021; 5, 284-294
3. Thomas Sr ED. Stem cell transplantation: past, present and future. Stem cells. 1994 Jan 1;12(6):539-44.

4. Hematopoietic Stem Cell Transplantation in Children and Adolescents Gregory M.T. GuilcherPediatrics in Review 2016;37;135
5. Fung, S. S., Elbaz, U., Mireskandari, K., & Ali, A. (2019). Graft versus host disease: a pediatric perspective. Expert Review of Ophthalmology, 14(2), 89–99.
6. Breviglieri CNM, Borges NMTF, Netto GZ, Gomes AA. Early complications in pediatric hematopoietic stem cell transplantation. J Bone Marrow Transplant Cell Ther. 2021;1(2):140-149.
7. Schmid I, Stachel D, PagelP .Incidence, Predisposing Factors, and Outcome of Engraftment Syndrome in Pediatric Allogeneic Stem Cell Transplant RecipientsBiology of Blood and Marrow Transplantation, 14, 438-444
8. Nishitani M, Graham RA, Wang T, Devos J, Lee SJ, Spellman SR, et al. Impact of age on graft-versus-host disease and overall survival in pediatric hematopoietic cell transplant recipients. Blood Adv. 2026;10(6):2108-2121.
9. Angelucci E, Matthes-Martin S, Baronciani D, Bernaudin F, Bonanomi S, Cappellini MD, et al. Hematopoietic stem cell transplantation in thalassemia major and sickle cell disease: indications and management recommendations from an international expert panel. Haematologica. 2014;99(5):811-820.
10. De Avila C, Martinez PA, Sendi P, Galvez Silva JR, Maher OM, Totapally BR. Hematopoietic Stem Cell Transplantation in Children with Sickle Cell Disease and Thalassemia Major: A National Database Study. PediatrHematolOncol. 2024 Oct;41(7):489-503.
11. Xiao H, Huang Q, Lai Y, Liu R. Haploidentical hematopoietic stem cell transplantation in pediatric transfusion-dependent thalassemia: a systematic review and meta-analysis. Transplant Cell Ther. 2025;31(2):101.e1-101.e12
12. Moiz, B., Habib, A., Sawani, S., Raheem, A., Hasan, B., &Gangwani, M. (2018). Anthropometric measurements in children having transfusion-dependent beta thalassemia. Hematology, 23(4), 248–252.
13. Ara UN, Chowdhury T, Akhter S, Sultana T, Torsa M. Growth parameters in children with transfusion-dependent thalassemia and their correlation with serum ferritin. DS (Child) H J. 2023;39(2):88-92.
14. Mirhosseini N, Shahar S, Ghayour-Mobarhan M, Kamaruddin NA, Banihashem A, Azadbakht L, et al. Factors affecting nutritional status among pediatric patients with transfusion-dependent beta thalassemia. Mediterr J NutrMetab. 2013;6(1):79-87.
15. Amalia J. Thalassemia and Nutritional Status in Children. Journal of Health and Dental Sciences. doi:10.54052/JHDS
16. Martynov I, Klima-Frysch J, Kluwe W, Engel C, Schoenberger J. Safety of tunneled central venous catheters in pediatric hematopoietic stem cell recipients with severe primary immunodeficiency diseases. PLoS One. 2020 May 15;15(5):e0233016.
17. Sheth V, Grisariu S, Avni B, Stepensky P, Ashkenazi M, Shapira MY, Or R. Fludarabine-based reduced toxicity yet myeloablative conditioning is effective and safe particularly in patients with high-risk thalassemia undergoing allogeneic transplantation. Pediatr Blood Cancer. 2018 Nov;65(11):e27312.
18. Zajac-Spychala O, Kampmeier S, Lehrnbecher T, Groll AH. Infectious Complications in Paediatric Haematopoetic Cell Transplantation for Acute Lymphoblastic Leukemia: Current Status. Front Pediatr. 2022 Feb 10;9:782530. doi: 10.3389/fped.2021.782530. PMID: 35223707; PMCID: PMC8866305.
19. Agholme MB, Dahllöf G, Törlén JK, Majorana A, Brennan MT, von Bültzingslöwen I, Tan PL, Hu S, Sim YF, Hong C. Incidence, severity, and temporal development of oral complications in pediatric allogeneic hematopoietic stem cell transplant patients - a multicenter study. Support Care Cancer. 2023 Nov 16;31(12):702.
20. Bognàr T, Bartelink IH, Egberts TCG, Rademaker CMA, Versluys AB, Slatter MA et al.Association Between the Magnitude of Intravenous Busulfan Exposure and Development of Hepatic Veno-Occlusive Disease in Children and Young Adults Undergoing Myeloablative Allogeneic Hematopoietic Cell Transplantation. Transplant Cell Ther. 2022 Apr;28(4):196-202
21. Kwon DH, Jung S, Lee EJ, Lee JY, Moon S, Lee JW, Chung NG, Cho B, Kim HK. Incidence and risk factors for early-onset hypertension after allogeneic hematopoietic stem cell transplantation in children. Korean Circ J. 2013 Dec;43(12):804-10.