19TH EDITION

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SBTMO

PROCEEDINGS OF XXIX ANNUAL MEETING OF BRAZILIAN SOCIETY OF BONE MARROW TRANSPLANTATION AND CELLULAR THERAPY

XXIX CONGRESSO SBTMO
BRASÍLIA 2025
TCH no centro da transformação!
XXVIII ENCONTRO MULTIDISCIPLINAR EM TCH

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Dear Colleagues,

We are honored to present the proceedings of our congress, which is taking place for the second time in Brasília, in the Federal District, with Dr Andressa Melo as President, Dr. Adriano Arantes as Vice President, and Dr Carmen Bonfim as Scientific Coordinator. This event traditionally seeks to bring together all professionals involved in bone marrow transplantation, providing comprehensive care for each patient and with a scientific program that encompasses this view of BMT.

Papers from several Brazilian teams are available, addressing key topics ranging from patient self-care to Cellular Therapy with CAR T cells, mesenchymal stem cells, and Natural Killer cells.

We hope you enjoy this material and that each paper in this edition will be a spark of hope in our daily lives.

Best regards,

Fernando Barroso Duarte
Nelson Hamersclak
Editors-in-Chief

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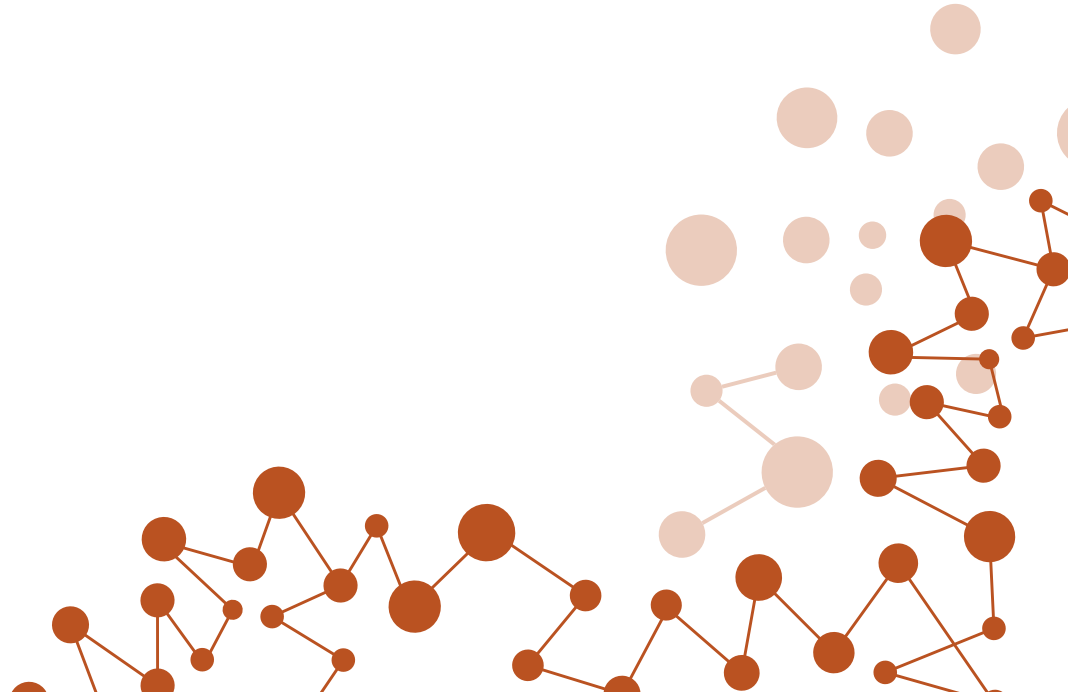

MARY FLOWERS AWARD

Best abstract in clinical aspects of HSCT

OUTCOMES OF HLA-MATCHED SIBLINGS, MISMATCHED, AND HAPLOIDENTICAL DONORS IN MYELODYSPLASTIC SYNDROMES: REPORT FROM THE LATIN AMERICAN REGISTRY.

Fernando Barroso Duarte[1], Yhasmine Delles Oliveira Garcia[2], Vaneuza Araújo Moreira Funke[3], Anderson João Simioni[4], Nelson Hamerschlak[5], Neysimélia Costa Villela[6], Maria Cristina Martins de Almeida Macedo[7], Afonso Celso Vigorito[8], Rodolfo Daniel de Almeida Soares[9], Alessandra Paz[10], Mariana Stevenazzi[11], Lilian Diaz[12], Abrahão Elias Hallack Neto[13], Gustavo Bettarello[14], Breno Moreno de Gusmão[15], Marco Aurélio Salvino[16], Rodolfo Froes Calixto[17], Maria Cláudia Rodrigues Moreira[18], Gustavo Machado Teixeira[19], Cinthya Corrêa da Silva[20], Eduardo José de Alencar Paton[21], Vanderson Rocha[22], Alicia Enrico[23], Carmem Bonfim[24], Ricardo Chiattone[25], Celso Arrais-Rodrigues[26], Erika Oliveira de Miranda Coelho[27], Marcelo Iastrebner[28], Beatrice Araújo Duarte[29], Vergílio Antônio Rensi Colturato[4].

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INTRODUCTION:

Allogeneic hematopoietic cell transplantation (HCT) remains the best curative option for myelodysplastic syndrome (MDS). Human leukocyte antigen (HLA)-matched sibling donors (MSD) are considered the preferred donor type in clinical practice. However, their availability is often below 30%, especially in older patients with MDS. For these patients, a matched unrelated donor (MUD) is considered a valid alternative but can take time to identify. Haploidentical (HID) HCT is an alternative in the absence of an HLA-matched donor. In general, clinical studies comparing recipient outcomes after HCT with HID versus MSD or MUD in myeloid malignancies have similar outcomes, with an overall survival (OS) between 40% and 80%, but few studies focused on the outcomes after allo-HCT for MDS patients.

OBJECTIVES:

The aim of this study is to analyze the characteristics and survival outcomes of patients undergoing HCT according to donor type.

METHODS:

This is a retrospective study with 448 patients from the transplant registry of 38 centers in Latin America (LA) available at tmo.med.br website, during 2016 to 2024. Patients were stratified according to donor type. Survival curves were performed using the Kaplan-Meier method, and log-rank test. The statistical program used was SPSS v.23.1 considering a significant $p<0.05$.

RESULTS:

The predominant donor type was MSD (62,72%) followed by MUD (24,11%) and HID (13,17%). Median recipient age at transplant slightly differed, with a higher median age of 46 (range 21) years in the MSD group ($p<0.001$). High/Very High risk R-IPSS category was more frequent in the HID group (p=0,014). Prior-treatment was more frequent in MUD and HID groups ($p<0.001$). Prior treatment with chemotherapy was more common among patients who received transplants from MSD (68.3%) and MUD (46.1%), whereas treatment with hypomethylating agents was more prevalent among patients who underwent transplantation from MUD (42.7%) and HID donors (63.8%) ($p<0.001$). Myeloablative conditioning was more frequent in MSD and MUD, whereas reduced-intensity conditioning was more common in HID (p=0.001). Chronic Graft Versus Host Disease (GVHD) was predominant in the MSD group (p=0,002). Post-transplant complications were observed in post HCT with similar frequency in the groups (p=0,17). The general frequency of death was 38.44%; however, it occurred at a similar rate across the donor groups (p=0.196). At 5-year, OS rate was 55.6% (CI 95% 50,30-61,40), with a median survival of 3.19 years (Figure 1). The OS estimate did not significantly differ according to donor type at 2 years (p=0.9) (Figure 2A) and at 5 years (p=0.74) (Figure 2B).

CONCLUSION:

The study showed no significant differences between donor types, indicating that despite the distinct characteristics of each group, donor type did not significantly impact survival or mortality after transplantation. Prospective studies in MDS are needed to confirm these results and clarify the issues raised.

KEYWORDS: allogeneic hematopoietic cell transplantation; myelodysplastic syndrome; donor type.

FIGURE 1: Overall survival (OS) after first allogeneic HCT from any donor type in patients with MDS (n=448).

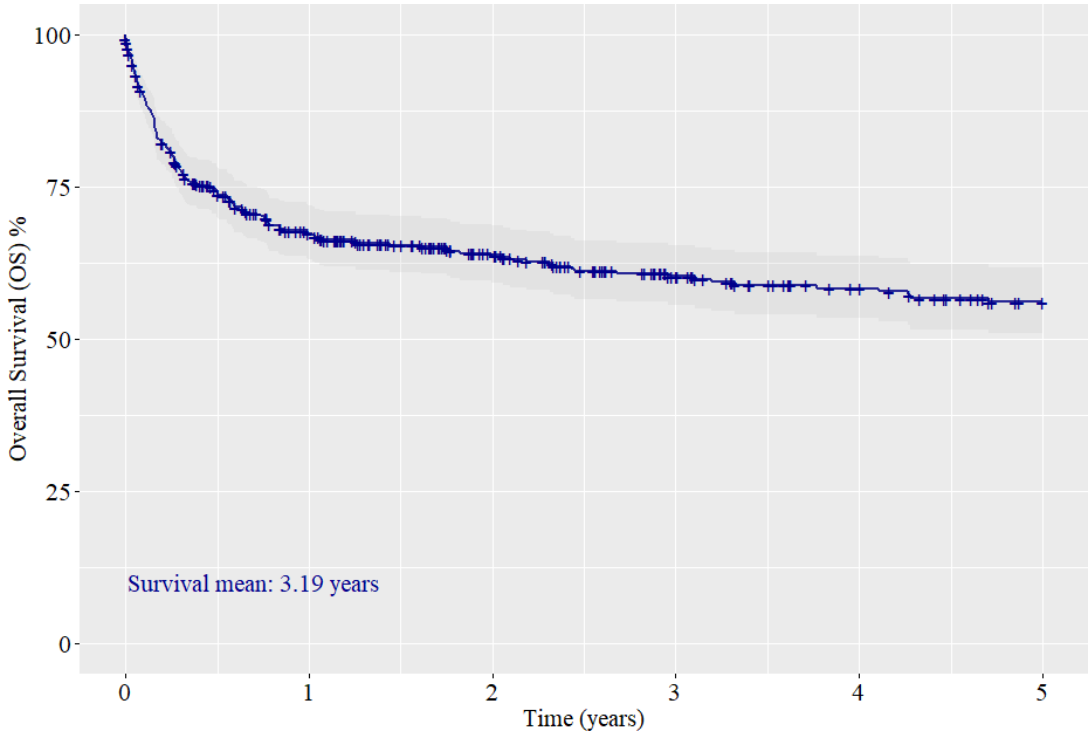
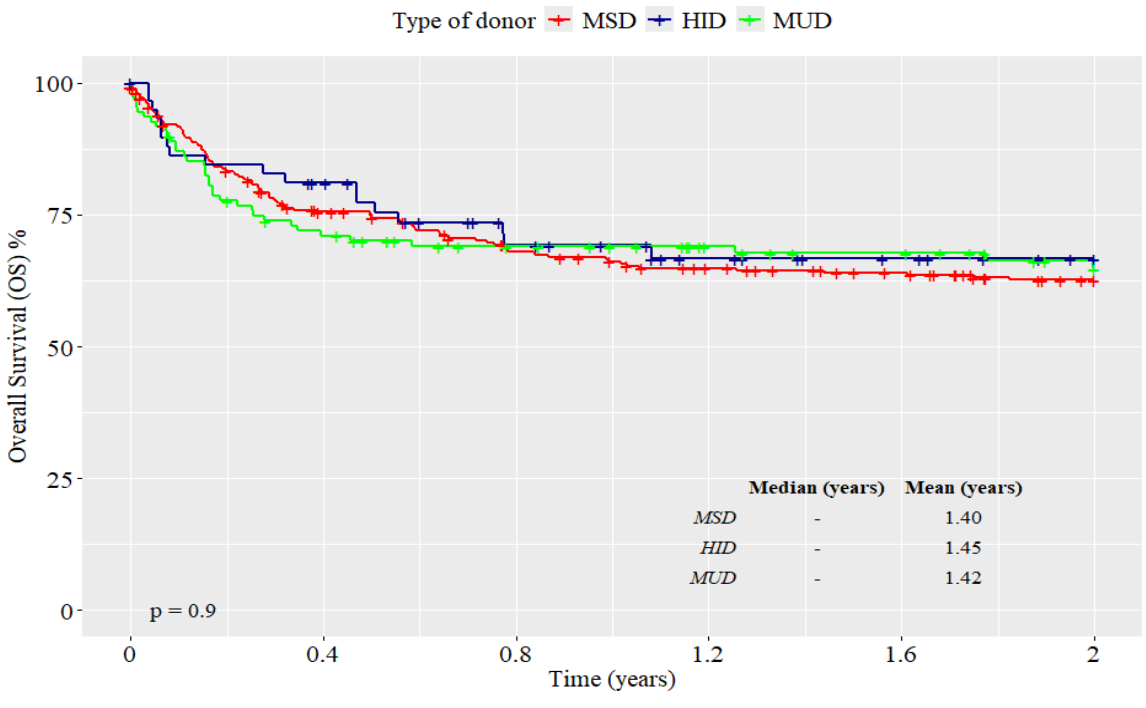


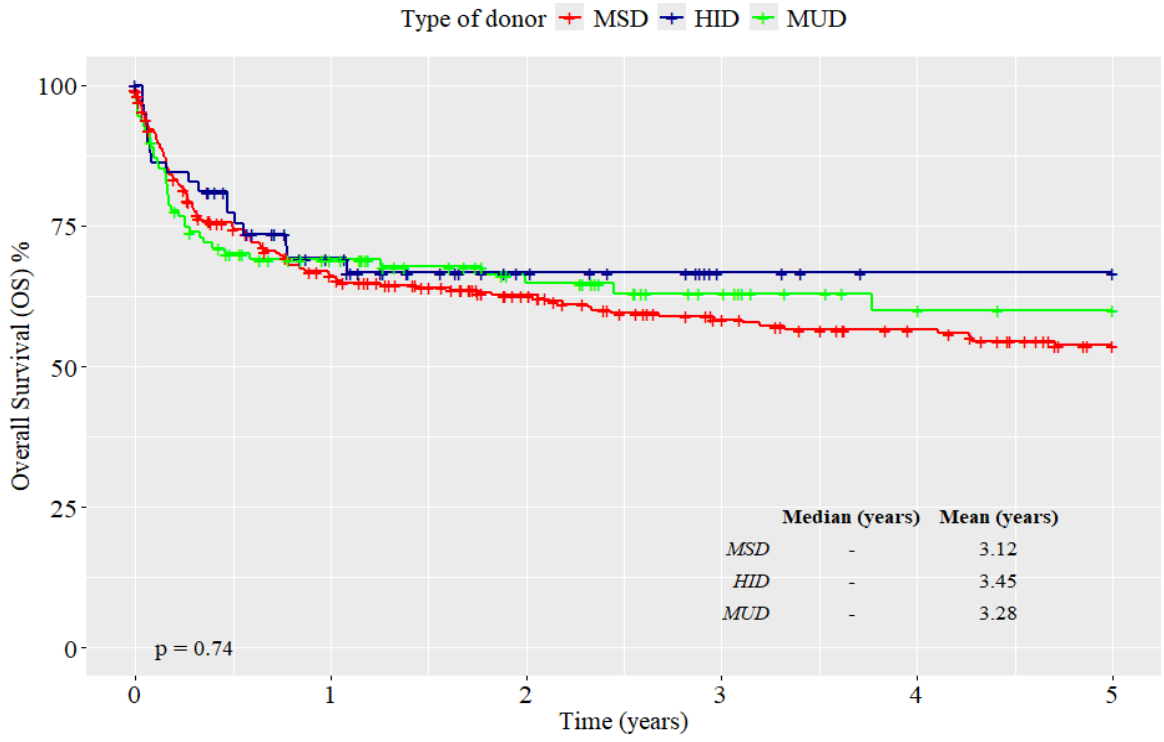
FIGURE 2: Overall survival (OS) after first allogeneic HCT in patients with MDS according to donor type (n=448). (A) Two-year OS estimates (p = 0.9); (B) Five-year overall survival estimates (p = 0.74).

A)



B)

B)


Type of donor MSD HID MUD
Overall Survival (OS) %
100
75
50
25
0
0
1
2
3
4
5
Time (years)
p = 0.74
Median (years) Mean (years)
MSD - 3.12
HID - 3.45
MUD - 3.28

JÚLIO VOLTARELLI AWARD

Best abstract in cell therapy and basic research

EXPANDING CAR-T CELL THERAPY IN BRAZIL: A DESCRIPTIVE ANALYSIS OF INDICATIONS FOR CHIMERIC ANTIGEN RECEPTOR T CELL THERAPY IN BRAZIL FROM 2020 TO APRIL 2025

Cinthya Corrêa da Silva[1], Anderson João Simione[2], Paula Moreira da Silva Sabaini[3], Antonio Vaz de Macedo[4], Heliz Regina Alves das Neves[5,6], Monique Ammi[7], Flavia Ferreira Costa[8], Celso Arrais Rodrigues da Silva[9], Adriana Mendes de Quadros Cavilha[5], Rosana Rocha Concilio[10], Phillip Scheinberg[10], Nelson Hamerschlak[1], Marco Aurelio Salvino[11], Adriana Seber [12,13,14], Yana Augusta Sarkis Novis[8], Vanderson Geraldo Rocha[15], Joaquim Gasparini dos Santos[15], Lúcia Silla[16,17], Ricardo Chiattone[12], Andreza Alice Feitosa Ribeiro[1], Vaneuza Araújo Moreira Funke[5,6], Leonardo Javier Arcuri[1,18], Lucila Nassif kerbauy[1], Jayr Schmidt Filho[19], Carmem Maria Sales Bonfim[20], Maria Claudia Rodrigues Moreira[21], Marcelo C. Pasquini[22] and Fernando Barroso Duarte[23,24]

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INTRODUCTION

Chimeric Antigen Receptor T cells (CAR-T cells) are genetically engineered cellular immunotherapies targeting antigens expressed on tumor cells. Initially approved for the treatment of acute lymphoblastic leukemia (ALL) and non-Hodgkin lymphoma (NHL), with excellent outcomes in advanced-stage patients, CAR-T cell therapy has since expanded to include multiple myeloma (MM) and Chronic lymphoblastic leukemia (CLL). The Brazilian Health Regulatory Agency (Anvisa) has approved four CAR- T cell therapies: Kymriah® (tisagenlecleucel) for ALL and NHL, Carvykti® (ciltacabtagene autoleucel) for MM, Yescarta® (axicabtagene ciloleucel) for NHL, and Tecartus® (brexucabtagen autoleucel) for ALL and mantle cell lymphoma. Anvisa mandates a 15-year follow-up for CAR-T cell recipients to monitor long-term safety, including secondary malignancies.

OBJECTIVE

To analyze and report the initial Brazilian outcomes of CAR-T cell therapy, based on data submitted to the Center for International Blood and Marrow Transplant Research (CIBMTR) from Brazilian centers, highlighting patient characteristics and treatment indications.

METHODS

Brazilian Centers for Advanced Cellular Therapy reported their data to the CIBMTR using the FormsNet3 electronic platform, a process protected by double authentication. The compiled, standardized, and coded data returned to Brazilian Society of Cell Therapy and Bone Marrow Transplantation (SBTMO) through the Data Back to Centers (DBtC) tool. Data reported between 2020 and April 2025 were extracted, and the spreadsheet was imported into Power BI Desktop (PBI). Functions were created to analyze the number of CAR- T cells, the number of centers, to translate columns into Portuguese, to categorize and classify diseases, and to group variables. Patient characteristics were detailed, including age, diagnosis, and type of CAR-T cell therapy (commercial vs. non-commercial). Data visualization was performed using Power BI.

RESULTS

Of the 104 CAR-T cell infusions, 86% (n=90) were in adults, with a median age of 56 years (range 4–83). The table shows the indication of CAR-T cell therapy over time. The primary indications were NHL (75 cases, 72%), ALL (23 cases, 22%), MM (4 cases, 4%) and CLL (2 cases, 2%) (Table 1). Commercial CAR-T cells accounted for 82% of infusions, with Kymriah® being the most frequently used product (63%). Most treatments occurred in São Paulo (75%). Non-commercial CAR-T cells were primarily used for NHL and ALL.

CONCLUSIONS

CAR-T cell therapy in Brazil is expanding, with increasing numbers of centers participating and reporting data to the CIBMTR. Ongoing data collection and long-term follow-up are crucial to understanding the full impact of these therapies. The partnership between the SBTMO and CIBMTR, along with Anvisa's regulatory framework, provides a robust infrastructure for monitoring and optimizing CAR-T cell therapy outcomes in Brazil.

KEYWORDS

CAR-T cells; cancer immunotherapy; chimeric antigen receptor (CAR); Data Management, CIBMTR, SBTMO

TABLE 1. No. of CAR-T cells infusions per year and according to the diagnosis

Disease	2020	2021	2022	2023	2024	2025*	Total
Non-Hodgkin lymphoma (NHL)	1	1	-	33	32	8	75
Acute lymphoblastic leukemia (ALL)	-	-	1	10	10	2	23
Multiple myeloma (MM)	-	-	3	-	-	1	4
Chronic lymphoblastic leukemia (CLL)	-	-	-	-	2	-	2
Total	1	1	4	43	44	11	104

*Data covers only from January to April.

FANI JOB AWARD

Best multidisciplinary abstract

CAN DENTAL INTERVENTION BE SAFE AFTER ALLO-HCT? INSIGHTS FROM A MULTICENTER STUDY ON SCALING AND ROOT PLANING

Maria Júlia Pagliarone[1], Mariana Andrade Costa[1], Lara Maria Alencar Ramos Innocentini[1], Tatiane Cristina Ferrari[1], Thalita Cristina de Mello Costa[1], Camila Campos Mesquita[1], Maria Elvira Pizzigatti Corrêa[2], Fernanda de Paula Eduardo[3], Letícia Mello Bezinelli[3], Nelson Hamerschlak[3], Héliton Spíndola Antunes[4], Maria Claudia Moreira[4], Simone Lermontov[4], Mary Evelyn Flowers[5], Leandro Dorigan de Macedo[1]

1 - Hospital of the Clinics of the School of Medicine of Ribeirão Preto
2 - State University of Campinas
3 - Hospital Israelita Albert Einstein
4 - Brazilian National Cancer Institute
5 - Fred Hutch Cancer Center

INTRODUCTION:

Scaling and root planing (SRP) is an effective intervention for reducing periodontal disease (PD) activity. PD-related inflammation is frequent in the post-allogeneic hematopoietic cell transplantation (allo-HCT). Although peripheral leukocyte counts recover numerically, immune reconstitution may remain incomplete for over a year. This period of immune immaturity increases susceptibility to infections. Invasive dental procedures, such as SRP, are known to cause transient bacteremia in immunocompetent individuals, but their safety in allo-HCT recipients is uncertain. As a result, such procedures are often contraindicated in the first post-transplant year. **Objective:** To evaluate the safety of SRP performed between days +90 and +130 following allo-HCT.

METHODS:

This multicenter, longitudinal, prospective, interventional, randomized study included allo-HCT recipients allocated into two groups: SRP (intervention) and observation only (control). Patients were randomized between days +90 and +130 post-transplant. All participants were monitored for 10 days for vital signs, infectious complications (bacteremia, sepsis, pneumonia), and antibiotic usage. Blood cultures were collected as follows: the control group had one sample at baseline (T0); the intervention group had samples at T0 (pre-SRP), T1 (2 hours post-SRP), and T2 (24 hours post-SRP). **Results**: A total of 216 patients were included (108 per group). Positive blood cultures at T0 were identified in 16 control patients (14.8%) and in 21 intervention patients (17.7%) across all time points. During the 10-day follow-up, 38 patients developed infectious complications, including 22 bacterial and 14 viral infections. Eight of these patients had a positive blood culture at T0. No significant differences were observed between groups regarding peripheral blood cultures at T0 ($p=0.98$), catheter blood cultures at T0 ($p=0.98$), infection rates ($p=0.47$), pneumonia ($p=0.5$), or antibiotic use ($p=1.00$). No cases of sepsis or bacteremia were reported in either group. Significant correlations were found between positive blood cultures at T0 and the occurrence of infection ($p<0.001$), and between peripheral and catheter blood cultures ($p<0.001$). **Conclusion:** SRP performed between D+90 and D+130 post-allo-HCT appears to be safe when supported by prophylactic antibiotics and clinical monitoring. These findings may inform future guidelines on dental care timing in immunocompromised populations.

KEYWORDS: allo-HCT, scaling and root planing, infection.

RICARDO PASQUINI AWARD

Young scientist best author abstract
with age equal or under 35

DYNAMICS OF INTESTINAL DOMINATION IN PATIENTS UNDERGOING ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION

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8. *Fundação Pio XII - Hospital de Câncer de Barretos, Barretos, Sao Paulo, Brazil.*

INTRODUCTION:

In patients undergoing allogeneic hematopoietic stem cell transplantation (allo-HSCT), an important prognostic factor is the dynamics of intestinal microbiota domination, which is a hallmark of intestinal dysbiosis, and is defined as the relative abundance of any single taxonomic unit exceeding 30%. Intestinal domination in international allo-HSCT studies can occur in up to 65% of patients, and it is usually due to the expansion of specific genera, including: 1) Enterococcus, 2) Bacteroides, 3) Blautia, 4) Lactobacillus and 5) Streptococcus. Despite being well-characterized in others allo-HSCT studies, the dynamics of intestinal domination in Brazilian patients remains poorly understood. Objective: In this study, using a cohort of Brazilian patients undergoing allo-HSCT, we aimed to identify the dynamics of intestinal microbiota domination.

METHODS:

This is a multicenter, observational prospective study, approved by the Research Ethical Committee. Subjects were patients >12 years old undergoing allo-HSCT. Fecal specimens were collected longitudinally at 7 time points: Prior to conditioning regimen (D-7); At the day of stem cell infusion (D0); 30 days after stem cell infusion (D+30); D+60, D+90, D+180, and at acute graft versus host disease diagnosis. Fecal DNA was extracted and 16S sequencing was performed by using Illumina platform. Bioinformatic analysis was performed, and the operational taxonomic units were used to determine the intestinal domination.

RESULTS:

During the study period, 69 patients provided 192 fecal specimens. Of these, 131 (68%) had intestinal domination. At least 42% of the samples collected at any time point had intestinal domination (see Figure 1). The highest prevalence of intestinal domination occurred at D+60 (n=29/30, 97%), while the lowest prevalence occurred in samples collected prior to the conditioning regimen (n=20/48, 42%). Most of these domination events (n=95/131; 73%) occurred due to expansion of four genera: 1) Bacteroides, 2) Akkermansia, 3) Phascolarctobacterium, and 4) Escherichia-Shigella (see Figure 2). Only one sample (0.5%) had Enterococcus domination.

CONCLUSION:

In Brazilian patients undergoing allo-HSCT, intestinal domination is a common event. However, the specific genera identified in our cohort are different from international studies. Future studies should assess if intestinal domination events by these specific genera are associated with worse clinical outcomes in Brazilian patients.

KEYWORDS: gastrointestinal microbiome; stem cell transplantation; prognosis.

FIGURE 1. Prevalence of intestinal domination by any genera during allo-HSCT.

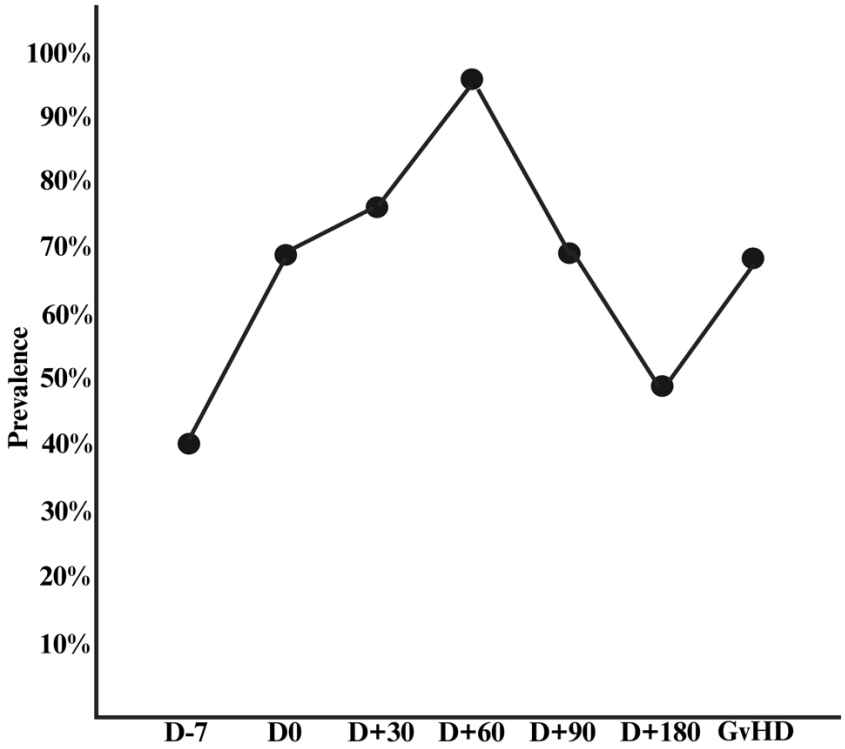
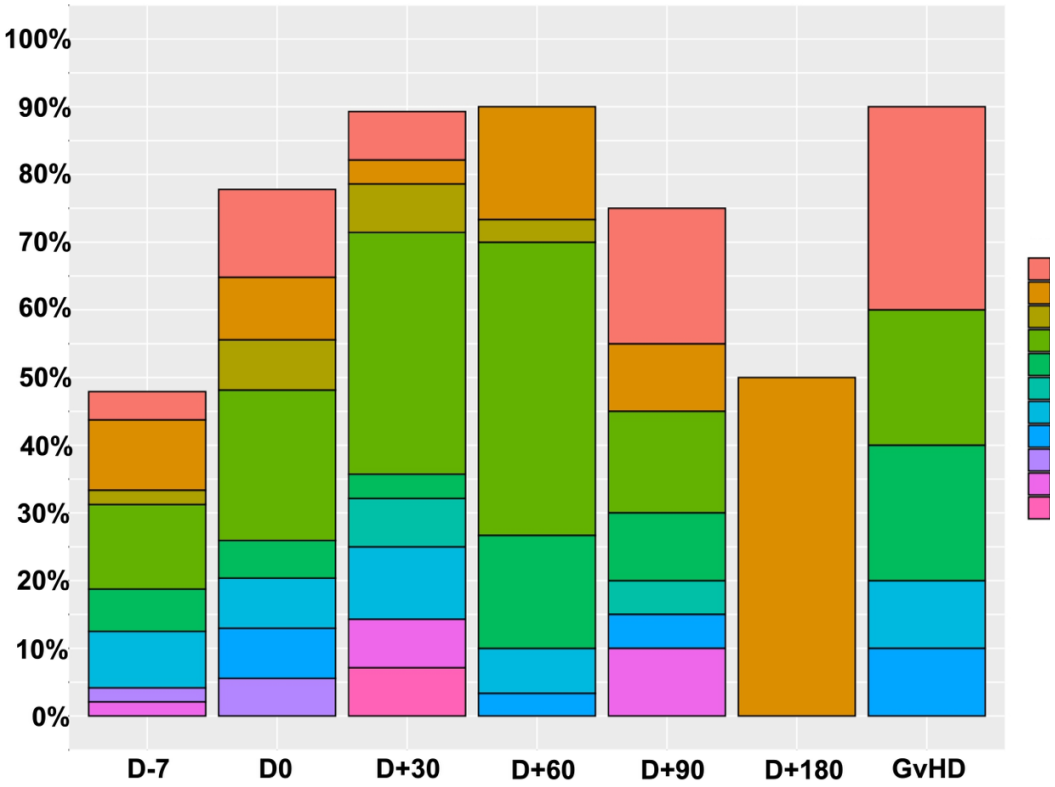


FIGURE 2. **Proportion of intestinal domination events by specific genera.**



CARMEM BONFIM AWARD

Best abstract in the pediatrics area

EASIX SCORE MAY BE A MARKER OF ENDOTHELIAL RISK AND HIGHER TRANSPLANT-RELATED MORTALITY IN PEDIATRIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR MALIGNANT DISEASES

Carla Nolasco Monteiro Breviglieri[1], Camila Noronha Santos[1], Valéria Cortez Ginani[1,2], Roseane Vasconcelos Gouveia[1,2], Gabriella Sayuri de Alencar[1], Milena Reis Santos de Oliveira[1,3], Anna Beatriz Willemes Batalha[1], Márcia Puato Vieira Pupim[1], Juliana Francielle Marques[1], Heloá Robbi[1], Adriana Seber[1,2]

1. Hospital Samaritano Higienópolis – São Paulo/SP
2. Instituto de Oncologia Pediátrica – GRAACC/UNIFESP – São Paulo/SP
3. Hospital da Criança de Brasília José Alencar – Brasília/DF

INTRODUCTION:

Endothelial dysfunction is a central driver of early transplant-related mortality (TRM) and morbidity, yet pediatric-specific risk stratification remains limited. The Endothelial Activation and Stress Index (EASIX), based on LDH x creatinine, divided by platelet count, has shown strong predictive value for sinusoidal obstruction syndrome (SOS), transplant-associated thrombotic microangiopathy (TA-TMA) and TRM in adult cohorts, but its utility in children is unvalidated. Despite its integration into ongoing international pediatric protocols, such as the FORUM 2 trial in acute lymphoblastic leukemia, no consensus yet exists on timing or thresholds for clinical use.

OBJECTIVE:

This study aimed to assess whether EASIX, measured at day 0 of hematopoietic stem cell transplantation, is associated with endothelial complications or early transplant-related mortality (TRM) in a pediatric population. **Methods:** We retrospectively analyzed 82 transplants in 80 pediatric patients. EASIX was calculated on the day of transplant. Endothelial complications were defined as sinusoidal obstruction syndrome (SOS), according to the revised pediatric EBMT criteria (Corbacioglu, 2018), and/or TA-TMA, as per the criteria proposed by Jodele (2014). TRM was defined as death up to day +100, excluding relapse. EASIX was analyzed as a continuous variable and categorized into three risk groups by distribution terciles: low (≤0.56), intermediate (0.57–1.30), and high (>1.30). Comparisons between groups were performed using Mann-Whitney and chi-square tests.

RESULTS:

Median age was 7 years (range 0–21). The median EASIX was 0.73 (range 0.09–9.54). Thirteen patients (16%) developed endothelial complications and three (3.7%) died from TRM. Median EASIX values were higher among patients with these complications (1.10 vs. 0.71, p=0.5) and in those who died from TRM (1.77 vs. 0.73, p=0.2), although differences did not reach statistical significance due to the low number of patients. When EASIX is categorized by terciles, SOS/TA-TMA and TRM rates were 11% in low, 14% in intermediate, and 23% in high EASIX scores and TRM occurred only with intermediate (n=1) and high (n=2) scores. In children with malignant diseases, a higher EASIX value was observed among those with complications (2.35 vs. 0.60; p = 0.056) and TRM (2.35 vs. 0.60; p = 0.056). This was not observed in non-malignant diseases or in recipients of autologous transplants, where the number of events was insufficient for meaningful comparisons.

CONCLUSIONS:

Collaborative prospective validation and measurement of the EASIX score in later time-points are critical to understand the role of this cheap and easy tool in the pediatric practice. Early EASIX assessment may assist in identifying high-risk children with malignant diseases who could benefit from closer monitoring. Future studies should explore serial measurements, to better capture the dynamic of the endothelial injury.

KEYWORDS:

Pediatric, EASIX, endothelial dysfunction

NELSON HAMERSCHLAK AND MARCELO PASQUINI AWARD

Best abstract in the data management area

FINE-TUNING OF PRE-TRAINED TRANSFORMER-BASED LARGE LANGUAGE MODELS FOR INFORMATION EXTRACTION FROM ELECTRONIC MEDICAL RECORDS

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INTRODUCTION:

Electronic medical records (EMRs) contain unstructured clinical data essential for research, clinical decision making, and outcome monitoring. However, extracting information from free-text remains challenging due to language variability. Recent advances in large language models (LLMs), particularly Transformer-based ones, have enabled significant progress in natural language understanding. This study explores fine-tuning pre-trained LLMs to extract key clinical variables - underlying disease, disease subtype, and diagnosis date - from EMRs in the context of hematopoietic cell transplantation (HCT).

OBJECTIVE:

To evaluate the performance of fine-tuned Transformer-based LLMs for extracting clinical information from Portuguese EMRs.

METHODS:

A total of 1258 EMRs written in Portuguese, from patients with hematological diseases undergoing HCT were used to build the dataset. Texts were normalized to lowercase, and special characters were removed. Each entry was annotated with three target variables: underlying disease, subtype, and diagnosis date. Missing values were labeled as NONE. Models training were conducted in Google Colab (NVIDIA A100-SXM4-40GB GPU) using the Transformers library (v4.51.3). Data was split into training (70%), validation (20%), and test (10%) sets. Five pre-trained T5-based models - T5-base, mT5-base, ptT5-base-v2, flanT5-base, and flanT5-large - were fine-tuned. The prompt used for training was: "Identify the underlying disease, disease subtype (if any), and date of diagnosis (which may correspond to bone marrow aspirate/biopsy date)." Training ran for 10 epochs (batch size 8). The models learning curves were evaluated using the loss function on the validation set. Models performance on the test set was evaluated based on the ROUGE-1 score, semantic accuracy (assessing the equivalence between generated and expected answers, from 0 to 1), and execution time per question (in seconds).

RESULTS:

All models demonstrated stable training with loss plateauing near epoch 8 (Figure 1). ROUGE-1 scores were near 1, showing high lexical and semantic fidelity (Table 1). Diagnosis date extraction had the lowest performance due to format inconsistency and presentation (Table 1), while disease and subtype extraction showed excellent results across models. mT5 showed the lowest semantic accuracy, while flanT5-large performed best. However, its improvement over flanT5-base was marginal and at the cost of triple execution time. Thus, flanT5-base emerged as the most efficient model for the task. **Conclusion:** Fine-tuned Transformer-based LLMs are effective for extracting clinical data from Portuguese EMRs, especially for identifying underlying diseases and subtypes. Although diagnosis date extraction remains more complex due to variability, overall results were robust. These models can significantly aid clinical research and decision-making by enabling scalable, automated information extraction from unstructured records.

KEYWORDS: Electronic Medical Records, Large Language Models, Transformer Models.

FIGURE 1: **Loss function curves of the models from epoch 3 onward.**

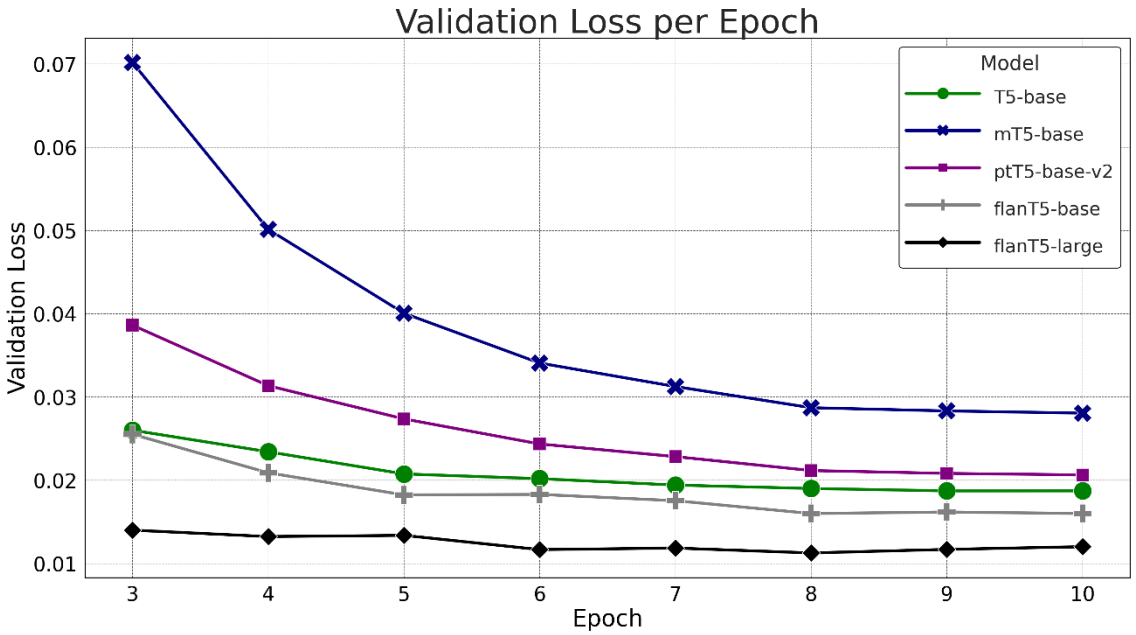

TABLE1. **Model performance metrics.**

Model	ROUGE-1 score	Semantic accuracy				Execution Time (seconds)
		Disease	Subtype	Diag. dt	Overall	
T5-base	0.94	0.95	0.91	0.75	0.87	0.98
mT5-base	0.92	0.84	0.86	0.73	0.81	0.86
ptT5-base-v2	0.95	0.94	0.89	0.78	0.87	0.79
flanT5-base	0.95	0.94	0.94	0.75	0.88	1.12
flanT5-large	0.95	0.96	0.93	0.78	0.89	3.39

ALIRIO PFIFFER AWARD

Best abstract in bone marrow failure syndromes

CLONAL EVOLUTION IN PAROXYSMAL NOCTURNAL HEMOGLOBINURIA WITH UNDERLYING BONE MARROW FAILURE: A LONGITUDINAL STUDY OF 44 PATIENTS

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INTRODUCTION:

Paroxysmal nocturnal hemoglobinuria (PNH) is a clinically heterogeneous disease, characterized by the presence of GPI-deficient clones that may expand or regress over time. Evaluating clonal kinetics may contribute to risk stratification and individualized therapeutic monitoring.

OBJECTIVE:

To assess the longitudinal variation in clonal size among patients with different clinical subtypes of PNH (Classic PNH, PNH/AA, SC-PNH). **Methods:** This study included 44 patients diagnosed with PNH between 2003 and 2021, comprising 10 cases of Classic PNH at diagnosis, 21 with bone marrow failure-associated PNH (PNH/AA), and 14 patients with subclinical PNH (SC-PNH). For each patient, the proportion of PNH clone was evaluated at two points by analyzing the loss of CD24 and FLAER markers (GPI-deficient population) in the neutrophil compartment. Paired statistical analysis was performed using the t-test and the Wilcoxon signed-rank test.

RESULTS:

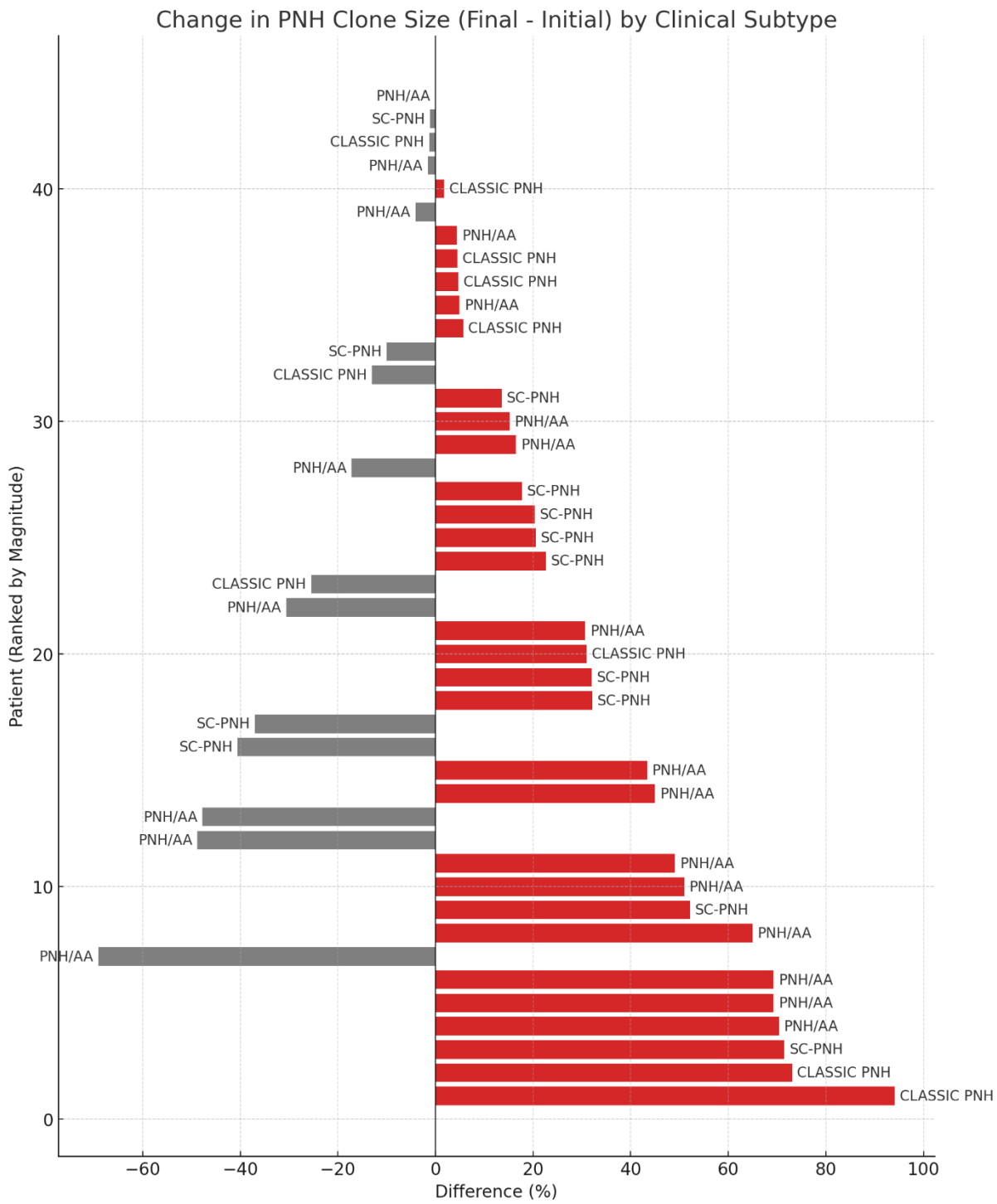
A reduction in clonal size was observed in 15 patients (33%), while 29 patients (66%) showed clonal expansion, with a mean variation of +15.5% (p = 0.01). A total of 37 patients received immunosuppressive therapy, two underwent hematopoietic stem cell transplantation (HSCT), and six patients with Classic PNH were treated with eculizumab as first-line therapy. Patients with clonal expansion accounted for 7/10 in Classic PNH (p = 0.18), 13/21 in PNH/AA (p = 0.07), and 9/13 in SC-PNH (p = 0.29). The PNH/AA subtype was the most represented among cases with greater clonal growth. Bar graph analysis revealed the largest clonal expansions in SC-PNH and PNH/AA subtypes, with one PNH/AA patient showing a +69.1% increase. Among patients with clonal reduction, two with Classic PNH became clone-negative following hematopoietic stem cell transplantation. The remaining 13 experienced gradual reduction, including six patients with a decrease greater than 30%. Patients with clonal evolution received immunosuppressive therapy, and six patients with Classic PNH were treated with eculizumab as first-line therapy.

CONCLUSION:

This analysis demonstrated a significant trend toward clonal expansion over time in the evaluated cohort, particularly in the PNH/AA and SC-PNH subtypes treated with immunosuppressive therapy. The high frequency of clonal growth in these groups underscores the importance of continuous monitoring of patients with Bone Marrow Failure.

KEYWORDS: bone marrow failure, Paroxysmal nocturnal hemoglobinuria, clonal evolution

FIGURE 1



Legend: Red bars indicate an increase in clone size (clonal expansion), and grey bars indicate a decrease in clone size (clonal reduction). Labels on each bar refer to the patient's PNH clinical subtype at diagnosis: CLASSIC PNH, PNH/AA: PNH associated with aplastic anemia or other marrow failure syndromes and SC-PNH (small clone, no symptoms).

ASTCT-SBTMO AWARD

Best abstract of young investigator

AN INTEGRATED SPATIAL MULTI-OMICS ANALYSIS OF CHRONIC ORAL GRAFT-VERSUS HOST DISEASE

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INTRODUCTION:

Minor salivary glands (MSGs) are emerging as a promising, accessible tissue source for early detection and monitoring of chronic graft-versus-host disease (cGVHD). Their anatomical location allows for longitudinal assessment of GVHD-related tissue damage. Histopathological changes often reflect systemic immune dysregulation and may occur before clinical symptoms manifest. Evidence suggests MSG alterations correlate with disease severity and can provide diagnostic and prognostic information. **Objective:** To identify key pathways, cellular interactions, and therapeutic targets involved in tissue remodeling.

METHODS:

This retrospective, observational study included 20 patients with confirmed histopathological diagnosis of chronic oral GVHD from 2014 to 2022. Biopsies were subjected to histology, immunohistochemistry, and spatial omics techniques, including spatial transcriptomics (Xenium) and spatial proteomics (Phenocycler Fusion). Techniques involved H&E staining, antibody panels, spatial gene expression, and multiplex protein analysis. Image analysis included segmentation and neighborhood evaluation. Advanced computational methods partitioned tissues, reconstructed cell-cell communication networks, and identified signaling patterns associated with immune infiltration and disease progression.

RESULTS:

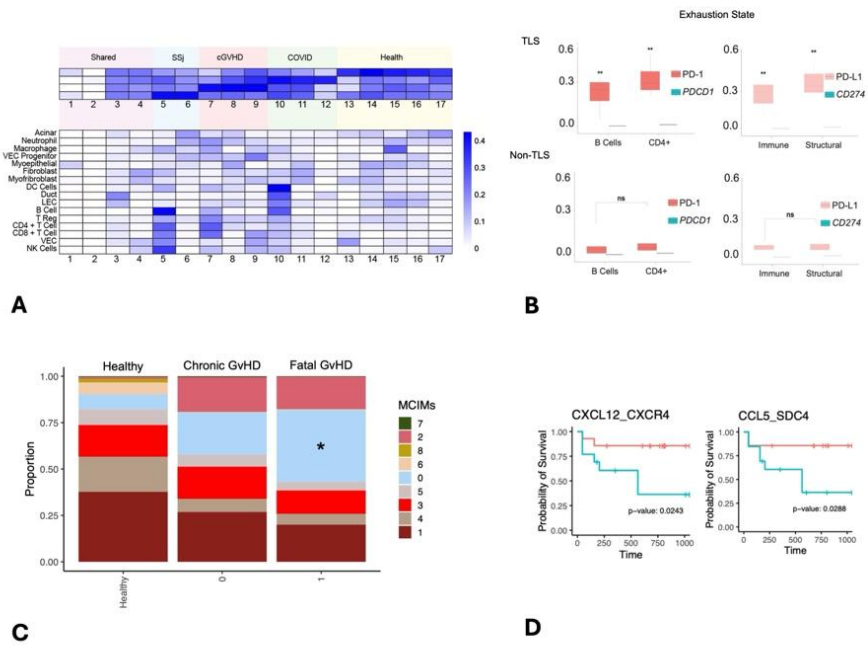
A total of 20 cases were studied; 55% were female, median age of 41 years. Acute leukemia and non-Hodgkin lymphoma were the most frequent underlying diseases. Most patients had mild severity and up to three organs involved at cGVHD onset (Table 1). In the MSG specimens, the most common changes were acinar atrophy(95%), periductal lymphocytic infiltrate(70%), and ductal metaplasia(65%). NK cells(CD56+) were statistically significantly correlated with stromal fibrosis(p=.01) and periductal stromal fibroplasia(p=.05). For

the first time, the periductal infiltration was characterized as Tertiary Lymphoid Structure (TLS)-like. Cell type distribution, exhaustion patterns, and chemokine interactions confirmed TLS formation(Fig. 1A). Integrated neighborhood analysis of "virtual" tissue microarrays across multiple salivary gland diseases revealed a distinct cellular distribution signature in cGVHD compared to healthy controls and other diseases (Fig. 1B). Fatal cGVHD displayed a unique proportion of multicellular interaction modules(receptor–ligand interaction patterns) compared to non-fatal cases(Fig. 1C). Among several receptor–ligand pairs, CXCL12–CXCR4 and CCL5–SDC4, transcribed by fibroblasts and pericytes, were significantly predictive of survival(Fig. 1D) **Conclusions:** In this cohort of cGVHD patients, MSGs biopsies revealed significant histopathological changes. Our multi-omics integrated analysis identified a distinctive cGVHD signature within multicellular interaction modules. Despite the small number of patients included, CXCL12-CXCR4 and CCL5-SDC4 axes were associated with survival and may be potential targets for therapeutic purposes.

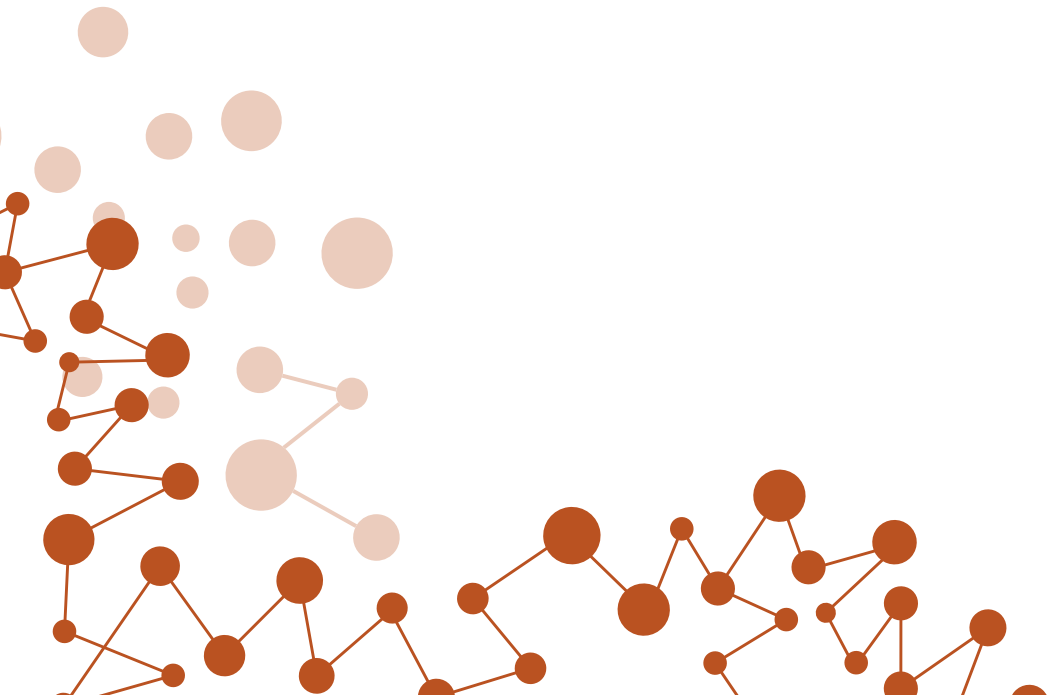
Table 1

Characteristic	N = 20[1]
Gender, N (%)	
Female	11 (55%)
Median age at HCT, years (IQR)	41 (29 - 53)
Disease type, N (%)	
Acute leukemia	7 (35%)
Non-Hodgkin Lymphoma	5 (25%)
CML	3 (15%)
Other	5 (25%)
HCT-CI, N (%)	
0-1	15 (79%)
≥ 2	4 (21%)
Unknown	1
Conditioning type, N (%)	
MAC	8 (50%)
RIC	8 (50%)
Unknown	4
GVHD prophylaxis, N (%)	
CsA + MTX	1 (33%)
PTCy + CsA + MMF	2 (67%)
Unknown	17
Donor type, N (%)	
Matched related	12 (71%)
Haploidentical	5 (29%)
Unknown	3
Cell Source, N (%)	
Peripheral blood	15 (83%)
Bone Marrow	3 (17%)
Unknown	2
Donor-recipient gender pairs, N (%)	
F→M	4 (22%)
Unknown	2
Maximum grade of acute GVHD, N (%)	
0-II	9 (82%)
III-IV	2 (18%)
Unknown	9
Global score of cGVHD at diagnosis, N (%)	
Mild	11 (73%)
Moderate/Severe	4 (27%)
Unknown	5
Time from HCT to diagnosis of cGVHD	
Median (IQR)	150 (110 - 312)
Unknown	2
Number of involved organs at diagnosis, N (%)	
1-3	11 (65%)
3	4 (24%)
≥ 4	2 (11%)
Unknown	3
Organ manifestations at diagnosis of cGVHD, N of affected patients (%)	
Mouth	9 (53%)
Skin	6 (35%)
Liver	5 (29%)
Eyes	4 (24%)
GI tract	4 (24%)
Lung	3 (18%)
Genital	2 (12%)
Unknown	3

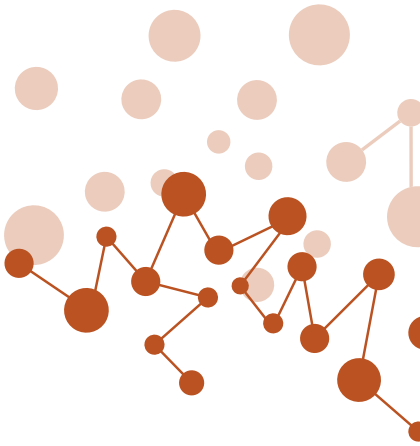
Figure 1



ORAL PRESENTATIONS



ALOGENEIC HSCT



ANALYSIS OF DONOR LYMPHOCYTE INFUSION (DLI) FOLLOWING ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION (ALLO-SCT) IN MYELOID MALIGNANCIES: A SINGLE-CENTER RETROSPECTIVE STUDY IN BRAZIL (2020–2025)

Luiz Frederico Bezerra Honorato Junior[1], Andrea Tiemi Kondo[1], Gabriela de Toledo Passos Candelaria[1], Andreza Alice Feitosa Ribeiro[1], Nelson Hamerschlak[1], Jose Mauro Kutner[1], Mariana Lorenzi Savioli[1]

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INTRODUCTION:

DLI represents a key strategy to enhance graft-versus-leukemia effects post allo-HSCT, as a prophylactic/preemptive or therapeutic strategy. However, its clinical benefit and risks remain under debate, particularly regarding timing, indication, and combination with systemic therapies.

OBJECTIVES:

To evaluate the indications, responses, and outcomes associated with different DLI strategies (therapeutic and prophylactic/preemptive) following allo-HSCT in patients with myeloid malignancies in a Brazilian transplant center from 2020 to 2025.

METHODS:

We conducted a retrospective, single center, analysis of patients who received at least one DLI following allo-HSCT for myeloid malignancies, including acute myeloid leukemia (AML), myelodysplastic syndrome (MDS), chronic myeloid leukemia (CML), and mixed phenotype acute leukemia. Collected clinical data included patient age, sex, pre-HSCT disease status, donor type (matched related donor, haploidentical donor, matched unrelated donor - URD, or mismatched URD), incidence of acute graft-versus-host disease (aGVHD) before and after DLI, response rates, time from allo-HSCT to first DLI, number of DLI infusions, and use of concomitant therapies. Patients were stratified according to DLI indication into therapeutic DLI and prophylactic/preemptive DLI groups. pDLI was defined as administration in the context of high-risk disease features, declining CD3+ donor chimerism, CD3+ chimerism <50%, or positive molecular minimal residual disease (MRD). Overall survival (OS) was estimated using Kaplan–Meier methodology and compared between groups using the log-rank test.

RESULTS:

During 2020 and 2025, a total of 30 patients were included, of whom 16 (53.3%) received tDLI and 14 (46.7%) received pDLI. The median number of DLI infusions was 2.4 (range: 1–6). The median time from allogeneic hematopoietic stem cell transplantation (allo-HSCT) to the first DLI was 224 days (range: 24–528) in the pDLI group and 420 days (range: 83–1744) in the tDLI group. All patients in the tDLI group received concomitant systemic therapy: 12 received hypomethylating agents (HMAs), 11 salvage chemotherapy, and 1 targeted therapy. In contrast, in the pDLI group, 9 patients received HMAs and 2 salvage chemotherapy. aGVHD of any grade pos-DLI was observed in 9 patients (30%); among them, 5 had grade I/II, 1 grade III, and 3 grade IV. Notably, all cases of grade IV aGVHD involved the gastrointestinal tract and occurred exclusively in the tDLI group. One-year OS was significantly higher in patients receiving pDLI compared to those receiving tDLI (82% vs. 36%, respectively; log-rank test, $p = 0.0087$), with a mean follow-up of 275 days after the first DLI.

CONCLUSION:

In this cohort, prophylactic/preemptive DLI was associated with improved overall survival and lower rates of severe GVHD compared to therapeutic DLI. These findings highlight the potential benefit of early intervention strategies in high-risk patients post allo-HSCT.

KEYWORDS: Donor lymphocyte infusion, bone marrow transplant, graft versus host disease.

Table 1. Baseline patientes characteristics

Characteristic	n (%)
Patientes	30
Male	11 (36.7%)
Female	19 (63.3%)
Median Age (Years)	50.7 (2–75)
Diagnosis	
AML	18 (60.0%)
MDS	8 (26.7%)
CML	2 (6.7%)
Biphenotypic Leukemia	1 (3.3%)
CMML	1 (3.3%)
Pre-HSCT Disease Status	
Refractory	6 (20.0%)
Partial Response	6 (20.0%)
Cytomorphologic Response	5 (16.7%)
MRD(+)	5 (16.7%)
MRD(-)	8 (26.7%)
Donor Type	
Matched Related Donor	8 (26.7%)
Matched Unrelated Donor (MUD)	11 (36.7%)
Mismatched Unrelated Donor (MMUD)	4 (13.3%)
Haploidentical	7 (23.3%)
Acute GVHD Before DLI	11 (36.7%)
Grade I	3 (10.0%)
Grade II	4 (10.0%)
Grade III	4 (13.3%)

Table 2. DLI characteristics

Characteristic	
DLI indication (Nº)	30
Therapeutic (tDLI)	16 (53,33%)
Preemptive/Prophylatic (pDLI)	14 (46.67%)
DLI associated therapy (Nº)	
Hypomethylating +/- Venetoclax +/- Ara-C	21
Targeted therapy	1
Chemotherapy	13
2º HSCT	2
Median-time from HSCT to first DLI (days)	328,6 (20-1744)
(tDLI)	224 (24-528)
(pDLI)	420 (83-1744)
Median number of DLIs (Nº)	2,4 (1-6)
Response Rates [Nº(%)]	
Progression	8 (26.7%)
Complete response	5 (16.67%)
MRD(+)	3 (10.0%)
MRD(-)	14 (46.67%)
Acute GVHD incidence (pos-DLI) [Nº(%)]	
Grade I	2 (6.7%)
Grade II	3 (10.0%)
Grade III	1 (3.3%)
Grade IV	3 (10.0%)

ANTI-THYMOCYTE IMMUNOGLOBULIN (THYMOGLOBULIN®) FOR GRAFT-VERSUS-HOST DISEASE PROPHYLAXIS IN MATCHED-RELATED DONOR PERIPHERAL BLOOD ALLOGENEIC HEMATOPOIETIC CELL TRANSPLANTATION

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2. Hospital Israelita Albert Einstein, Sao Paulo - SP - Brasil.

INTRODUCTION:

Graft-versus-host disease (GVHD) is the leading complication of allogeneic hematopoietic cell transplantation, significantly impairing quality of life. The peripheral blood stem cell (PBSC) source is one of the most recognized risk factors. Kroger et al. (PMID: 26735993) conducted a randomized trial demonstrating improved outcomes with antihuman T-lymphocyte immune globulin (ATLG) from a matched-related donor (MRD) using PBSC grafts. Unfortunately, they used the ALTG Fresenius brand, while Thymoglobulin is the only brand available in Brazil.

OBJECTIVE:

To report the outcomes of ATG Thymoglobulin-based PBSC in MRD and to compare our findings with those obtained by Kroger et al using ATLG.

METHODS:

This single-center cohort study included patients with hematologic malignancies who received MRD HCT using a PBSC graft from 2019 to 2024. Patients received a total ATG 4 mg/kg dose close to the HCT, in conjunction with a calcineurin inhibitor and an antimetabolite agent. Survival and cumulative incidence curves were constructed using the Kaplan-Meier or Gray method, respectively.

RESULTS:

With a median follow-up of 29 months, we included 36 patients. The median age was 45 years, and most patients had acute leukemia (Table 1). Two-year overall survival (OS) was 71%; progression-free survival (PFS), 71%; relapse, 17%; non-relapse mortality (NRM), 11%; chronic GVHD (cGVHD), 48%; and moderate or severe chronic GVHD (cGVHDms), 29% (Table 2). Six-month grades II-IV and III-IV acute GVHD (aGVHD) occurred in 36% and 8% of patients, respectively.

DISCUSSION:

We have observed impressive OS, PFS, relapse, and NRM rates with ATG Thymoglobulin in MRD utilizing PBSCs. The cGVHD rate was 48%, which falls between the 36% observed in the ALTG arm and nearly 70% in the control arm of the Kroger et al study. Similarly, our moderate or severe cGVHD rate was 29%, again situated between the 8% and 52% noted in the ALTG and control arms of the Kroger et al trial. Notably, the Kroger trial protocol commenced tapering cyclosporine around day +120 and ceased immunosuppression around day +180, whereas in our cohort, the calcineurin inhibitor is typically discontinued before day +180.

CONCLUSION:

we have demonstrated excellent results with Thymoglobulin in MRD HCT using PBSC, partially confirming the results of the Kroger et al trial with ATG-Fresenius. The following step involves evaluating the effects of stopping calcineurin inhibitors early and the impact of either prophylactic or preemptive donor lymphocyte infusion in our cohort.

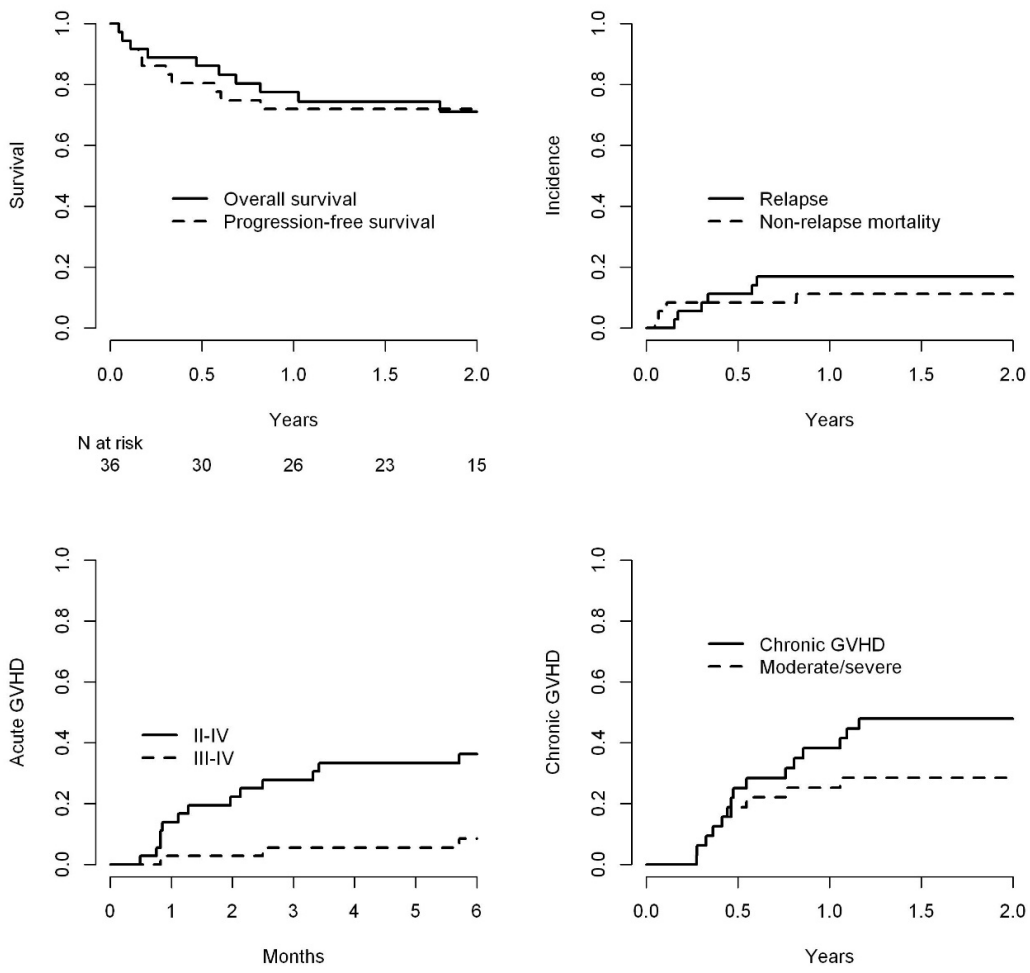
TABLE 1. Patients' profile

Variable	N
Total	36
Mean age (SD)	45 (16)
Female sex	22 (61%)
Primary disease	
Ambiguous lineage leukemia	1 (3%)
Acute lymphoblastic leukemia	11 (31%)
Acute myeloid leukemia	13 (36%)
Chronic myeloid leukemia	5 (14%)
Hodgkin lymphoma	1 (3%)
Non-Hodgkin lymphoma	2 (6%)
Myelodysplastic syndrome	3 (8%)
Median follow-up (IQR)	25 (23-38)

TABLE 2. Outcomes

Outcome	Est.	CI
2-year overall survival	71%	57-88%
2-year progression-free survival	72%	59-88%
2-year relapse	17%	8-35%
2-year non-relapse mortality	11%	4-28%
6-month aGVHD, II-IV	36%	23-56%
6-month aGVHD, III-IV	8%	3-25%
2-year cGVHD	48%	33-69%
2-year cGVHD, mod/sev	29%	16-50%

FIGURE 1. **Selected outcomes**



JOINT USE OF RABBIT ATG AND POSTTRANSPLANT IMMUNOSUPPRESION WITH CYCLOPHOSPHAMIDE FOR HLA-MATCHED PERIPHERAL BLOOD HEMATOPOIETIC CELL TRANSPLANTATION – THE NCT06299462 JURASSIC PHASE I/II TRIAL

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INTRODUCTION

A randomized phase III trial has demonstrated the feasibility of sole posttransplant cyclophosphamide (PTCy) in HLA-matched hematopoietic cell transplantation (HCT) with bone marrow grafts. However, this approach results in unacceptable rates of graft-versus-host disease (GVHD) when the graft source is peripheral blood stem cells (PBSC). We hypothesized that incorporating low-dose ATG would effectively prevent GVHD.

OBJECTIVE

To evaluate the feasibility of HLA-matched PBSC HCT using only ATG and PTCy prophylaxis, without calcineurin inhibitors or antimetabolite agents for GVHD prevention.

METHODS

This prospective, interventional, unicenter phase I/II trial was registered at ClinicalTrials.gov under NCT06299462. The inclusion criteria were age between 18-60 years; acute leukemia in CR1 or CR2, myelodysplasia with < 20% blasts, or lymphoma; HLA 8/8-matched donor; and PBSC. The only exclusion criterion was liver dysfunction (renal dysfunction was not excluded). Recruiting began in June 2024. Patients received ATG 4-5 mg/kg, proximal to the HCT, with PTCy at 50 mg/kg on D+3 and D+4. The CD34 cell dose was capped at 5e6 CD34/kg. No other agents for GVHD prophylaxis were allowed.

RESULTS

With a median follow-up of 6 months, ten patients were included. Patients' profiles are in Table 1. Sixty percent received a myeloablative conditioning regimen, while 40% underwent reduced-intensity. All patients engrafted, although one had a secondary graft failure, in the context of other unexplained and currently under investigation graft failures in our service. 40% had grades 3-4 cytokine release syndrome (CRS) early post-infusion. Three had acute GVHD disease grade II, two after donor lymphocyte infusion (DLI) for immune reconstitution for refractory grade 4 BK-virus cystitis or measurable residual disease, and another patient following a protocol violation (it received 6.7e6 CD34/kg). Except for one patient who died from mesenteric thrombosis, all patients are alive. One patient who received DLI was diagnosed with moderate, steroid-sensitive chronic GVHD. Only one patient, with refractory Hodgkin lymphoma previously exposed to 7 lines of therapy, had disease progression. 6-month overall and progression-free survivals were 100% and 83%, respectively (Figure 1). Seven (70%) patients had clinically significant CMV reactivation, with no cases of CMV disease. The median CD4 at 6 months was 225/mm3 (interquartile range: 186-244).

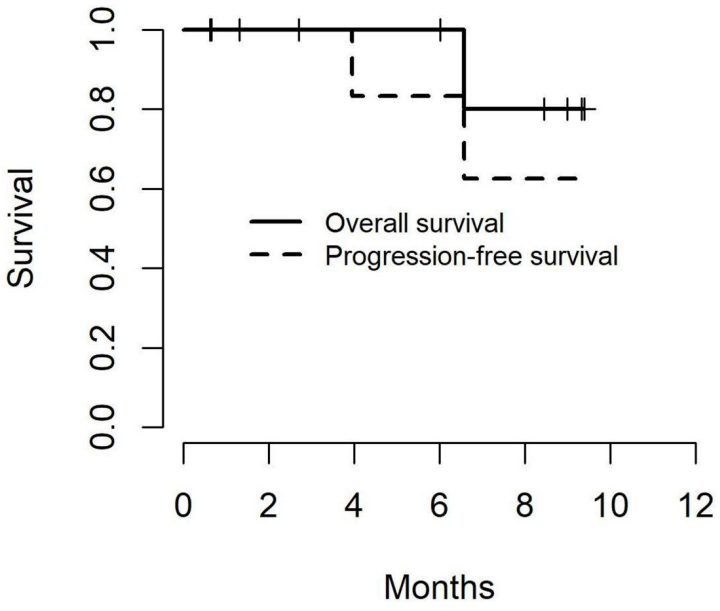
DISCUSSION

With a short follow-up, we show the feasibility and promising results of the novel ATG and PTCy-only GVHD prophylaxis. GVHD incidence was low, and no patient had the severe forms of acute GVHD. Adequate immune reconstitution at 6 months was noted. The next step will be to include a short-course immunosuppression to prevent CRS. We plan to move forward shortly to phase II of the trial.

Table 1. Patients' characteristics

Variable	N = 10
Female sex	4 (40%)
Median HCT-CI	0.5 (0-3)
Primary disease	
Acute myeloid leukemia	2 (20%)
Acute lymphoblastic leukemia	4 (40%)
Hodgkin lymphoma	4 (40%)
DRI	
Low	2 (20%)
Intermediate	5 (50%)
High	3 (30%)
Recipient CMV+	10 (100%)
Donor CMV+	8 (80%)
Donor	
MSD	9 (90%)
MUD	1 (10%)
Female donor sex	3 (30%)
Conditioning	
RIC, FluMel	4 (40%)
MAC, BuFlu	2 (20%)
MAC, FluTBI	2 (20%)
MAC, CyTBI	2 (20%)
Mean CD34, e6 (SD)	4.9 (0.8)
Mean CD3, e6 (SD)	196.9 (95.9)

Figure 1. Overall and progression-free survival



IMPACT OF A LONG-TERM FOLLOW-UP PROGRAM ON ADHERENCE TO SCREENING AND PREVENTION GUIDELINES IN SURVIVORS OF ALLOGENEIC BONE MARROW TRANSPLANTATION

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BACKGROUND:

Allogeneic hematopoietic cell transplantation (HCT) is increasingly used to treat both malignant and non-malignant conditions, emphasizing the need for effective long-term follow-up strategies. In 2019, our Cell Therapy Program established a Long-Term Follow-Up Program (LTFUP) to systematically address post-HCT complications.

OBJECTIVE:

To assess the adherence to the 2011 screening and prevention guidelines (Majhail et al., BBMT 2012; 18(3):348-71) before and after the LTFUP implementation.

METHODS:

This retrospective cohort study compared two groups of allogeneic HCT survivors: a pre-LTFUP group (2014–2016) and a post-LTFUP group (2019–2021). Eligible patients were over 18 years old, survived at least two years post-HCT, and experienced no disease relapse or progression. Adherence to the 2011 consensus guidelines for screening and preventive practices was assessed within two years post-HCT for both cohorts. Both physician and patient adherence were evaluated.

RESULTS:

Of the 86 allo-HCTs performed between 2014 and 2016, 35 met inclusion criteria; from 40 HCTs performed between 2019 and 2021, 28 were included. Most patients were male (58%), with a median age of 37–40 years. The most common diagnosis was acute leukemia, and HLA-related donors were predominant in both groups. Post-LTFUP, there was a significant increase in the use of peripheral blood as a graft source (from 54% to 86%, p=0.008) and in the use of post-transplant cyclophosphamide for GVHD prophylaxis (from 17% to 50%, p=0.005). Regarding physician adherence, the post-LTFUP group showed significantly better adherence to glycated hemoglobin testing (p<0.001) and microalbuminuria screening (p<0.001), with an increased frequency of repeat testing for microalbuminuria (p=0.004). Pharmacological interventions for lipid management were also more frequent after program implementation (p=0.002). Additionally, referrals for skin cancer screening, dental evaluations, and ophthalmologic assessments were significantly higher in the post-LTFUP group (p<0.001) (Table 1). Patient adherence was high, with only three of 86 patients not fully compliant with recommended work-up. No baseline characteristic was associated with increased patient adherence to screening or preventive measures.

CONCLUSION:

Despite the limited sample size, establishing a structured LTFUP at our center appears to have improved adherence to key recommendations outlined in the 2011 guidelines. These findings underscore the importance of dedicated follow-up programs in enhancing post-transplant care and ensuring better long-term health outcomes in HCT recipients. Further research is necessary to confirm these results and evaluate the impact on clinical outcomes.

Table 1. Adherence to key tests, evaluations and interventions

Characteristic	Overall N = 63	Pre-LTFUP N = 35	LTFUP N = 28	p-value
Bone Densitometry				0.6
Adherent	60 (95%)	34 (97%)	26 (93%)	
Non-Adherent	3 (5%)	1 (3%)	2 (7%)	
Vitamin D				>0.9
Adherent	62 (98%)	34 (97%)	28 (100%)	
Non-Adherent	1 (2%)	1 (3%)	0 (0%)	
Lipid panel				0.5
Adherent	61 (97%)	33 (94%)	28 (100%)	
Non-Adherent	2 (3%)	2 (6%)	0 (0%)	
HgbA1c level				**<0.001**
Adherent	46 (73%)	18 (51%)	28 (100%)	
Non-Adherent	17 (27%)	17 (49%)	0 (0%)	
Fasting glucose				>0.9
Adherent	62 (98%)	34 (97%)	28 (100%)	
Non-Adherent	1 (2%)	1 (3%)	0 (0%)	
Thyroid function				>0.9
Adherent	62 (98%)	34 (97%)	28 (100%)	
Non-Adherent	1 (2%)	1 (3%)	0 (0%)	
Microalbuminuria				**<0.001**
Adherent	25 (40%)	7 (20%)	18 (64%)	
Non-Adherent	38 (60%)	28 (80%)	10 (36%)	

Characteristic	Overall N = 63	Pre-LTFUP N = 35	LTFUP N = 28	p-value
Mammography				0.6
Adherent	14 (74%)	8 (67%)	6 (86%)	
Non-Adherent	5 (26%)	4 (33%)	1 (14%)	
Not applicable	44	23	21	
Pap test/Cervical cancer screening				0.4
Adherent	18 (72%)	9 (67%)	9 (86%)	
Non-Adherent	7 (28%)	5 (33%)	2 (14%)	
Not applicable	37	21	17	
Unknown	1	1	0	
Dermatologic evaluation				**<0.001**
Adherent	41 (65%)	16 (46%)	25 (89%)	
Non-Adherent	22 (35%)	19 (54%)	3 (11%)	
Dental evaluation				**<0.001**
Adherent	46 (73%)	19 (54%)	27 (96%)	
Non-Adherent	17 (27%)	16 (46%)	1 (3.6%)	
Ophthalmic evaluation				**<0.001**
Adherent	45 (71%)	18 (51%)	27 (96%)	
Non-Adherent	18 (29%)	17 (49%)	1 (3.6%)	

IMPROVEMENT OF SIGNS AND SYMPTOMS OF VULVOVAGINAL CHRONIC GRAFT VERSUS HOST DISEASE WITH NON-ABLATIVE RADIOFREQUENCY THERAPY

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INTRODUCTION:

Chronic graft-versus-host disease (cGVHD) is a major cause of late morbidity and mortality following allogeneic hematopoietic cell transplantation (allo-HCT). Gynecological complications of allo-HCT include vulvovaginal cGVHD, premature ovarian insufficiency, and genital atrophy. Non-ablative radiofrequency (NARF) therapy has shown beneficial effects in managing genital atrophy. However there are no published reports evaluating its use in vulvovaginal manifestations of chronic GVHD.

OBJECTIVE:

To report a case series of female patients with genital cGVHD treated with NARF therapy.

METHODS:

Data were retrospectively collected from electronic medical records of female patients who underwent allo-HCT from 2005 to 2023. Inclusion criteria were a diagnosis of cGVHD based on the 2014 National Institutes of Health (NIH) Consensus Criteria, with documented vulvovaginal involvement, with or without associated atrophy. Eligible patients received at least three sessions of NARF therapy between January 2019 and June 2024. Treatments were performed using the Tonederm® device, with sessions spaced 30 to 45 days apart. Target temperatures were 40–45°C for the vaginal canal and 35°C for the vulvar region.

RESULTS:

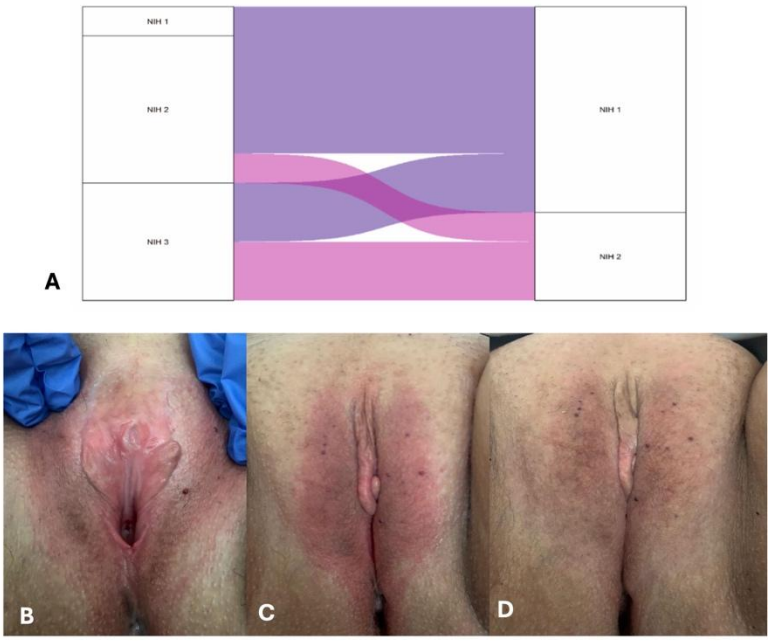
Ten patients were included, with a median age of 42 years (range: 37–51). Nine patients received grafts from HLA-identical sibling donors, and one from a matched unrelated donor. Peripheral blood was the most common graft source (8/10), followed by bone marrow (2/10). Conditioning intensity was myeloablative in 6 patients, reduced-intensity in 3, and unknown in 1. GVHD prophylaxis consisted of a calcineurin inhibitor and methotrexate in 9 cases, while one patient received post-transplant cyclophosphamide-based prophylaxis. At the time of the first NARF session, NIH genital scores were as follows: 1 in 1 patient, 2 in 5 patients, and 3 in 4 patients. Nine patients underwent three NARF sessions; one patient received four. Six out of nine patients were on concomitant systemic immunosuppressive therapy, and all patients received concurrent topical hormonal therapy.After three or four sessions of NARF therapy: 5 patients improved from NIH score 2 to 1, 2 patients improved from score 3 to 2, 2 patients improved from score 3 to 1, 1 patient maintained a score of 2 (see Figure 1). Adverse events included one case of localized thermal injury during the final session and one case of genital herpes simplex virus reactivation. No other significant side effects attributable to the therapy were observed.

CONCLUSION:

Despite the small number of patients, NARF therapy appears to be a promising supportive treatment for vulvovaginal cGVHD. Prospective studies are warranted to further evaluate its safety and therapeutic efficacy in this context.

KEYWORDS: Non-ablative radiofrequency, Vulvovaginal, chronic graft versus host disease

FIGURE 1: A) Genital NIH score before and after non-ablative radiofrequency; B-D) Evolution of the vulvovaginal aspect in a patient with genital cGVHD after one (B), two (C), and three (D) sessions of non-ablative radiofrequency therapy.



INITIAL CALCINEURIN INHIBITOR LEVELS AND EARLY ACUTE KIDNEY INJURY IN HEMATOPOIETIC CELL TRANSPLANT: A RETROSPECTIVE SINGLE-CENTER STUDY OF 634 RECIPIENTS

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INTRODUCTION:

Acute kidney injury (AKI) is a frequent and severe complication after hematopoietic cell transplantation (HCT), contributing significantly to post-transplant morbidity and mortality. Among several contributing factors, the nephrotoxicity of calcineurin inhibitors (CNI), widely used for graft-versus-host disease (GVHD) prophylaxis, plays a critical role. Therefore, understanding the impact of initial plasma levels of these drugs may help in early risk stratification and optimization of immunosuppressive management.

OBJECTIVE:

To evaluate the association between the initial plasma level of CNI and the incidence of AKI during the first three weeks after CNI initiation in HCT recipients.

METHODS:

This was a retrospective, single-center study including HCT recipients from 2002 to 2023. AKI was classified according to KDIGO 2012 criteria, based on serum creatinine levels, into stages 1, 2, and 3. Baseline creatinine was defined as the last stable value prior to CNI initiation, maintained stable for ≥5 days. AKI was assessed during the 1st, 2nd, and 3rd weeks, as well as across the consolidated day 1 to 21 (D1–21) period. The initial plasma level (IPL) was defined as the first available measurement after initiation of cyclosporine (CSA) or tacrolimus (TAC), categorized as therapeutic (CSA: 200–300 mcg/L; TAC: 5–15 ng/mL), subtherapeutic (<200/<5), or supratherapeutic (>300/>15). Descriptive analyses of clinical and transplant-related variables were performed. Associations between AKI and clinical variables were assessed using the chi-square test, followed by univariate logistic regression to analyze the association between IPL category and AKI incidence, with emphasis on combined AKI stages 2–3.

RESULTS:

A total of 634 patients were included, with 563 (88.8%) receiving CSA and 71 (11.2%) receiving TAC. The overall incidence of AKI within the first 21 days was 46.8% (stage 1: 16.4%, stage 2: 21.9%, stage 3: 8.5%). AKI occurred in 48% of CSA patients and 38% of TAC

patients (p = 0.114). The occurrence of AKI (any stage) was not significantly associated with transplant- or patient-related variables such as transplant type, stem cell source, underlying disease, ethnicity, ABO compatibility, sex, TBI, or presence of acute GVHD. However, when considering only stages 2–3, the presence of GVHD was associated with a lower incidence of AKI (25.0% vs. 34.7%; p = 0.010). Non-therapeutic IPLs—particularly supratherapeutic—were associated with an increased risk of AKI across multiple time points, especially during the second week, the D1–21 interval, and in more severe AKI stages (Table 1).

CONCLUSION:

Initial plasma CNI levels significantly impact the incidence of AKI in the first three weeks after initiation of immunosuppression. Supratherapeutic levels were associated with higher AKI risk, particularly in more severe cases, underscoring the importance of achieving therapeutic CNI levels early to improve clinical outcomes.

KEYWORDS:

Calcineurin Inhibitors; Acute Kidney Injury; Hematopoietic Cell Transplantation

TABLE 1. Association Between Initial Plasma Calcineurin Inhibitor Levels and the Incidence of Acute Kidney Injury Within the First 21 Days

1st plasma level	AKI (1st W)		AKI (2nd W)		AKI (3rd W)		AKI (1-21D)		AKI Stage 2-3 (1-21D)	
	OR (CI)	P	OR (CI)	P	OR (CI)	P	OR (CI)	P	OR (CI)	P
Therapeutic	0.76 (0.46-1.23)	0.271	0.51 (0.34-0.79)	0.002	0.89 (0.61-1.29)	0.542	0.68 (0.48-0.97)	0.034	0.87 (0.60-1.27)	0.493
Non-therapeutic	1.31 (0.80-2.14)		1.92 (1.26-2.94)		1.12 (0.77-1.62)		1.45 (1.02-2.05)		1.14 (0.78-1.66)	
Infratherapeutic	0.48 (0.28-0.49)	0.005	0.74 (0.49-1.11)	0.155	0.82 (0.55-1.23)	0.353	0.60 (0.41-0.87)	0.008	0.59 (0.39-0.88)	0.011
Supratherapeutic	2.08 (1.25-3.46)		1.34 (0.89-2.01)		1.20 (0.81-1.79)		1.65 (1.13-2.40)		1.68 (1.12-2.52)	

AKI Acute kidney Injury; OR odds ratio; CI confidence interval; P P value for univariate logistic regression; W week; D Days.

OUTCOMES OF ALLOGENEIC HEMATOPOIETIC CELL TRANSPLANTATION FOR HYPOPLASTIC MYELODYSPLASTIC SYNDROME: A LATIN AMERICAN MULTICENTER ANALYSIS

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BACKGROUND:

Myelodysplastic Syndromes (MDS) are a heterogeneous group of blood neoplasias characterized by cytopenia, myeloid dysplasia, and increased risk of progression to acute myeloid leukemia. The hypocellular-MDS (h-MDS), a rare subtype accounting for 10–15% of MDS patients, that is defined by reduction of ≤25% bone marrow (BM) cellularity, age adjusted according to WHO-2022. These patients are typically younger and characterized by more severe cytopenias, higher transfusion dependence and lower blast percentages as compared to normo/hypercellular MDS (n-MDS). The aim of this study is to to evaluate the clinical characteristics and overall survival (OS) of patients with h-MDS compared to those with normo-/hypercellular MDS (nMDS), based on data from the Latin American Registry.

METHODS:

This is a retrospective study with 458 patients undergoing HCT at diagnosis. Clinical data were obtained from the transplant registry of 38 centers in Latin America (LA) between January 2000 to June 2025. In the study, of patients were qualified for the diagnosis of MDS-h based on WHO-2022. Survival curves were performed the Kaplan-Meier method, and log-rank test. The statistical program used was SPSS v.23.1 $p<0.05$ were considered statistically significant.

RESULTS:

Among a total of 458 patients, 69 (15.07%) were diagnosed with h-MDS, while 389 (84.93%) had n-MDS. Based on bone marrow cellularity, the median age was 52 years in the h-MDS cohort and 57 years in the n-MDS cohort. In the h-MDS group, 39 patients (56,52%) were male, with a mean age of 57.0 ± 20.3 years, According to the IPSS-R, (53.85%) of patients were classified as intermediate risk, (25.64%) as low/very low risk, (15.38%) as high risk, and (5.13%) as very high risk. Most received grafts from compatible related donors (62,32%) and peripheral blood stem cells (55,07%) using myeloablative conditioning (68,12%). Death occurred in 31.88% of patients with MDS-h. Post-transplant complications were observed in (75.36%) of patients. The main causes of complications were acute GVHD (50%) (Table 1). Significant differences were observed between h-MDS and n-MDS groups. Patients <65 years, IPSS-R Low and Intermediate risk categories (p=0.017), prior chemotherapy (p=0.030), and use of reduced-intensity conditioning regimens (p=0.030) were more frequent in the h-MDS group. Additionally, h-MDS patients showed a significantly lower relapse rate compared to n-MDS (p=0.023). No statistically significant difference in overall survival (OS) was observed between patients with h-MDS and n-MDS undergoing allo-HCT, both at 5 years (HR=0.66; 95% CI: 0.42–1.04; p=0.07) (Figure 1a) and at 20 years (p=0.095) (Figure 1b). At 5-years, progression-free survival (PFS) patients with h-MDS had a significantly lower risk of relapse or death compared to n-MDS after allo-HCT (HZ=0,63; (CI 95% = 0,40 - 0,99); p = 0,046.) (Figure 2). The Non-relapse mortality (NRM) at 5 years after allo-HCT did not differ significantly between patients with h-MDS and n-MDS HZ=0,82;(95% CI=0,51-1,30); p=0.392 (Figure 3). Among h-MDS patients, IPSS-R very high-risk was associated with an increased risk of mortality in the univariate analysis (HR=2.18; 95% CI: 1.33–3.59; p=0.002). In the multivariate analysis, male sex (HR=1.36; 95% CI: 1.00–1.85; p=0.047) and IPSS-R very high-risk (HR=2.85; 95% CI: 1.69–4.81; p<0.001) remained significant independent predictors of worse survival (Table 2).

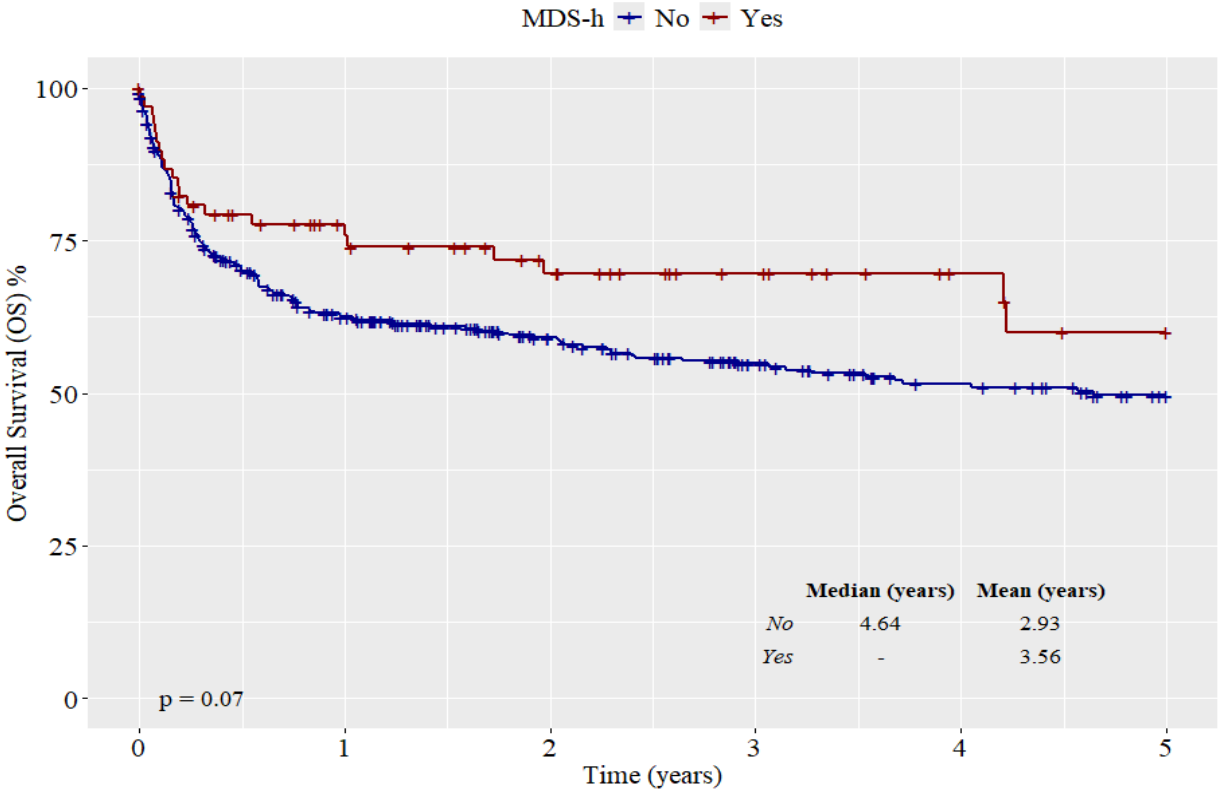
CONCLUSION:

We found that h-MDS was associated with distinct clinicobiological characteristics, as evidenced by significant differences in survival and IPSS-R risk category distribution. These findings reinforce the biological heterogeneity between subgroups and its impact on prognosis, which may influence decisions regarding HCT.

KEYWORDS: Hematopoietic cell transplantation; Myelodysplastic Syndromes; Hypocellular, Latin America Registry

FIGURE 1: Overall survival (OS) at 5 and 20 years after allogeneic HCT in patients with h-MDS and n-MDS.

a)



b)

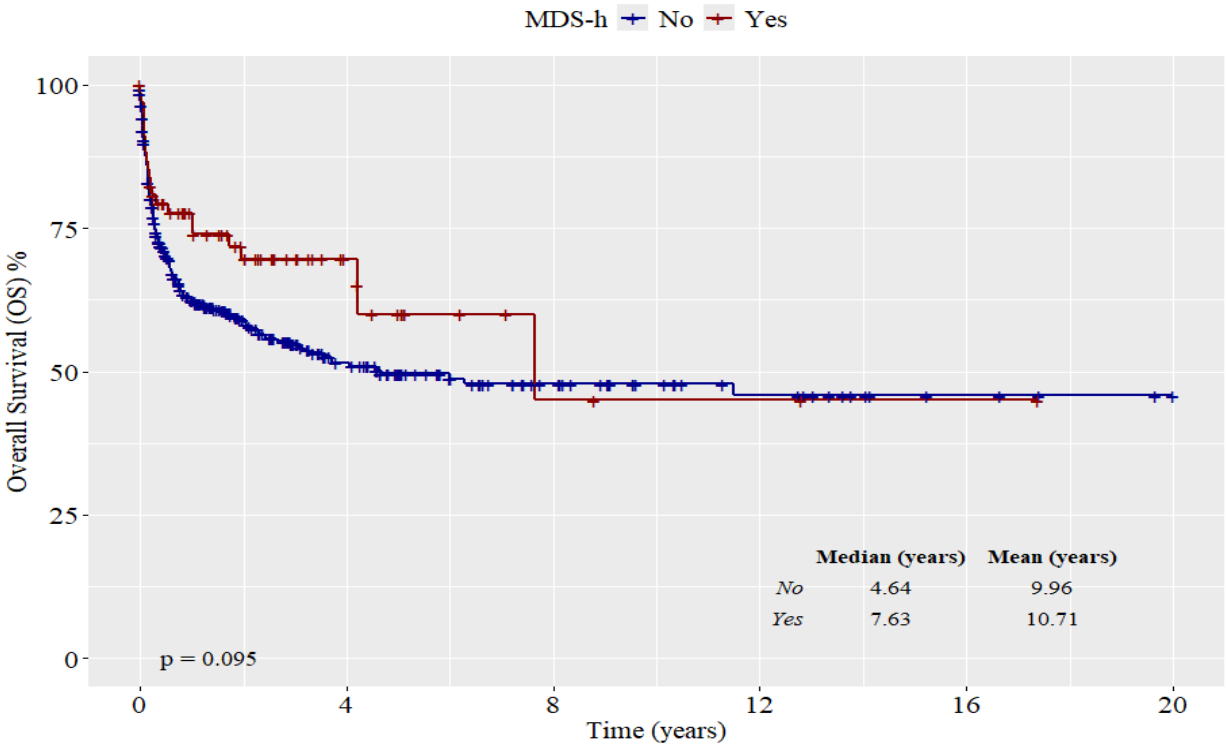


FIGURE 2: Progression Free-Survival (PFS) at 5 Years After Allo-HCT in Patients with (h-MDS) and (n-MDS).

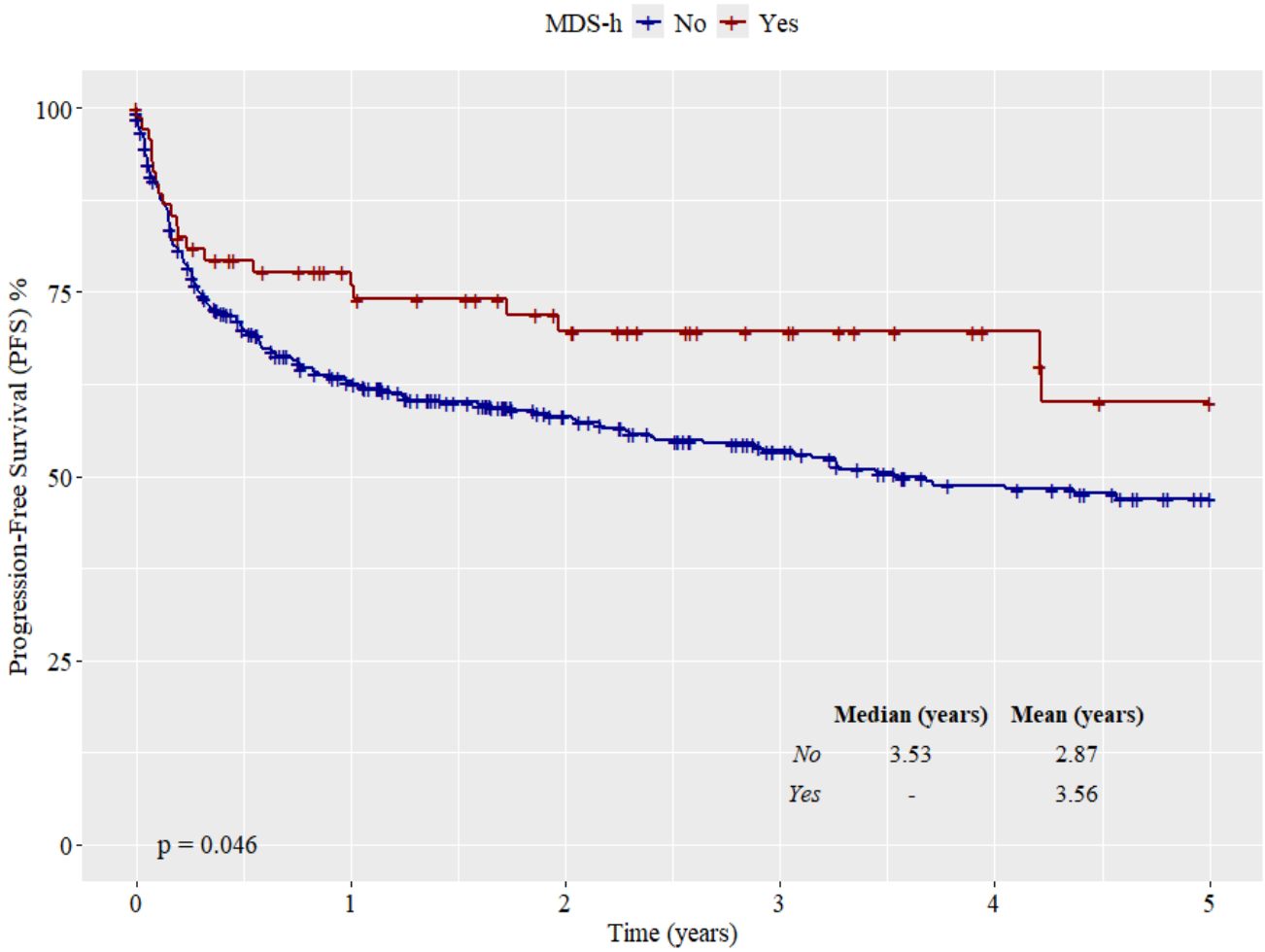


FIGURE 3: **Non-relapse mortality (NRM) at 5 Years After Allo-HCT in Patients with (h-MDS) and (n-MDS).**

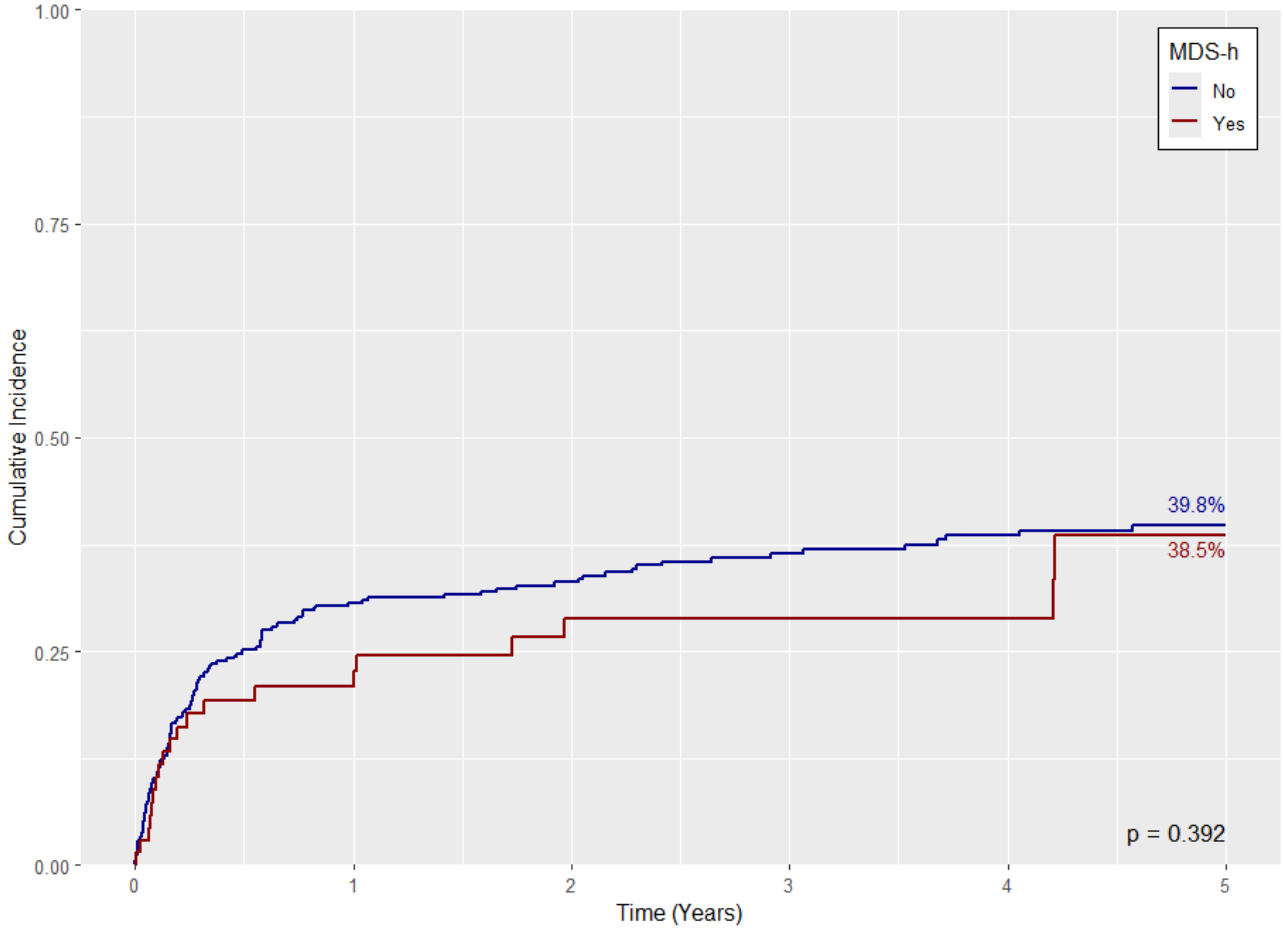


TABELA 1. Patient characteristics (N=458).

Variable MDS-h (n = 69)	Group MDS-n (n = 389)	p	
Patient age at transplant, median (range), year	52 (1-80)	57 (1- 85)	
Age Group			
< 65 anos	63 (92,65%)	316 (81,87%)	0,042 a
65 years or older	5 (7,35%)	70 (18,13%)	
Gender			
Female	30 (43,48%)	165 (42,42%)	0,974 a
Male	39 (56,52%)	224 (57,58%)	
IPSS-R b			
Very Low Risk	0 (0%)	2 (0,87%)	0,017b
Low	10 (25,64%)	31 (13,54%)	
Intermediate	21 (53,85%)	81 (35,37%)	
High Risk	6 (15,38%)	84 (36,68%)	
Very High Risk	2 (5,13%)	31 (13,54%)	
Previous Treatment			
Yes	41 (59,42%)	272 (70,83%)	0,081 a
No	28 (40,58%)	112 (29,17%)	

Type of Previous Treatment			
Chemotherapy	32 (46,38%)	137 (35,68%)	0,003 a
Hypomethylating agents	8 (11,59%)	100 (26,04%)	
Chemotherapy + Hypomethylating	1 (1,45%)	35 (9,11%)	
Missing data	28 (40,58%)	112 (29,17%)	
Conditioning regimen			
Reduced-intensity	22 (31,88%)	99 (25,45%)	0,030b
Myeloablative	47 (68,12%)	263 (67,61%)	
Non-Myeloablative	0 (0%)	27 (6,94%)	
Donor type			
Related	43 (62,32%)	243 (62,63%)	0,880 a
Haploidentical	8 (11,59%)	52 (13,4%)	
Unrelated	18 (26,09%)	93 (23,97%)	
Graft source			
Umbilical cord placental blood	2 (2,9%)	4 (1,03%)	0,201b
Bone marrow	29 (42,03%)	190 (48,97%)	
Mobilized blood cells	38 (55,07%)	194 (50%)	

Death			
Yes	22 (31,88%)	172 (44,22%)	0,075 a
No	47 (68,12%)	217 (55,78%)	
Post-transplant Complications			
Yes	52 (75,36%)	305 (78,41%)	0,686 a
No	17 (24,64%)	84 (21,59%)	
Chronic GVHD			
Yes	15 (28,85%)	108 (35,41%)	0,446 a
No	37 (71,15%)	197 (64,59%)	
Acute GVHD			
Yes	26 (50%)	137 (44,92%)	0,597 a
No	26 (50%)	168 (55,08%)	
Relapse			
Yes	1 (1,75%)	44 (13,21%)	0,023a
No	56 (98,25%)	289 (86,79%)	

Note: p-value a. Fisher Exact Test b: Chi-Square Test.*Bacterial infections, including multidrug-resistant bacterial infections, and fungal infections. Abbreviations: CMV:cytomegalovirus; GVHD: graft-versus-host disease; HLA: Human Leucocyte Antigen; HCT: Hematopoietic Cell Transplantation; R-IPSS: Revised International Prognostic Scoring System.

TABLE 2: Univariate and Multivariate Cox Regression Models for Overall Survival MDS-h.

Variables	Univariate Model			Multivariate Model		
	HR	95% CI	p value	HR	95% CI	p value
Gender						
Female	1			1		
Male	1,29	(0,96–1,74)	0,086	1,36	(1,00–1,85)	0,047
Age Group						
< 65 anos	1			1		
65 years or older	1,11	(0,77–1,60)	0,572	1,14	(0,76–1,71)	0,536
R-IPSS						
Very Low Risk	1			1		
Low Risk	0,94	(0,54–1,64)	0,821	0,93	(0,53–1,63)	0,796
Intermediate	1,13	(0,78–1,65)	0,524	1,1	(0,75–1,62)	0,617
High Risk	1,18	(0,80–1,75)	0,393	1,22	(0,82–1,81)	0,332
Very High Risk	2,18	(1,33–3,59)	0,002	2,85	(1,69–4,81)	<0,001
Previous Treatment						
Yes	1			1		
No	0,9	(0,67–1,22)	0,508	0,83	(0,60–1,15)	0,252
Conditioning regimen						
Reduced-intensity / Non-Myeloablative	1			1		
Myeloablative	1,14	(0,84–1,56)	0,399	1,32	(0,92–1,88)	0,127
MDS Type						
Others	1			1		
MDS-h	0,66	(0,42–1,04)	0,072	0,65	(0,41–1,04)	0,070
Donor type						
Related	1			1		
Haploidentical	0,71	(0,43–1,17)	0,178	0,76	(0,45–1,27)	0,296
Unrelated	0,85	(0,60–1,21)	0,373	0,93	(0,65–1,34)	0,700

PERIPHERAL BLOOD VERSUS BONE MARROW GRAFT SOURCES FOR POSTTRANSPLANT CYCLOPHOSPHAMIDE-BASED MATCHED AND MISMATCHED UNRELATED DONOR TRANSPLANTS

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INTRODUCTION

Peripheral blood grafts (PB) are recognized as a risk factor for graft-versus-host disease (GVHD) in nearly all scenarios when compared to bone marrow (BM) in hematopoietic cell transplantation (HCT). However, the effect of PB relative to BM remains unclear with PTCy-based GVHD prophylaxis involving matched and mismatched unrelated donors. Objective

Compare PB with BM in patients undergoing HLA 8/8 (MUD) or HLA 7/8 (MMUD) unrelated HCT with PTCy-based prophylaxis.

METHODS

This cohort, registry-based study included patients between 2017 and 2021 with acute myeloid leukemia, acute lymphoblastic leukemia, or myelodysplastic syndrome who underwent HCT with MUD or MMUD and PTCy. Main outcomes were relapse-free survival (RFS) and moderate or severe chronic GVHD (cGVHDms) incidences, measured as hazard ratios. We hypothesized that RFS would not differ and that cGVHDms would be higher with PB. We utilized a Center for International Blood and Marrow Transplant Research (CIBMTR) registry data. Kaplan-Meier and cumulative incidence curves were compared with the logrank and Gray tests, respectively. Multivariable Cox models were chosen based on the lowest AIC. Acute GVHD outcomes were reported at 6 months, while all others were reported at 24 months.

RESULTS

With a median follow-up of 31 months, 1554 and 127 received PB or BM from MUD, while 511 and 102 received PB or BM from MMUD, respectively. Patients' characteristics were well balanced, except for myeloablative conditioning regimen (more likely with BM) and sirolimus (more likely with BM in MMUD). Uni and multivariable analyses are in Table 1. In the MMUD group, PB and BM RFS (HR = 0.91, 95%CI 0.66-1.26, p = 0.57) and cGVHDms (HR = 1.24, 95%CI 0.70-2.19, p = 0.47) also revealed no significant differences. When we combined both groups (MUD and MMUD) for the analyses, we found no differences between PB and BM for RFS. Other outcomes (overall survival, relapse, non-relapse mortality, grades II-IV acute GVHD, and grades III-IV acute GVHD) were also similar in all comparisons.

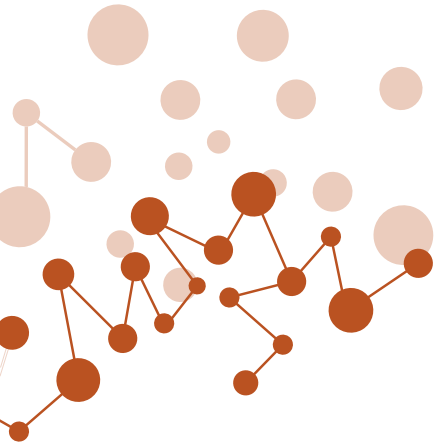
DISCUSSION

In a large CIBMTR database, our results demonstrate no substantial difference in any outcomes between BM and PB in MUD or MMUD transplants with PTCy-based GVHD prophylaxis. These findings were quite surprising, as PB has long been recognized as a risk factor for GVHD. Until now, a decision paradox existed: a significantly more comfortable donation process associated with PB has been previously reported, while BM achieved better patient results. It suggests PB should be the preferred graft source for MUD or MMUD with PTCy-based prophylaxis without adverse effects for the recipient.

Table 1. Outcomes

Outcome	BM	95%CI	PB	95%CI	p	HR*	95%CI	p
HLA 8/8								
OS	62%	54-71	65%	63-68	0.75	0.87	0.65-1.18	0.37
RFS	54%	46-63	57%	55-60	0.53	0.86	0.66-1.12	0.26
REL	28%	21-38	26%	24-29	0.60	0.90	0.64-1.26	0.53
NRM	18%	12-26	16%	15-18	0.87	0.83	0.54-1.29	0.41
II-IV aGVHD	32%	25-41	29%	27-32	0.61	1.04	0.75-1.45	0.81
III-IV aGVHD	6%	3-13	6%	5-8	0.91	0.93	0.45-1.91	0.84
Mod/sev cGVHD	6%	3-12	11%	9-13	0.06	1.59	0.74-3.43	0.24
HLA 7/8								
OS	64%	55-74	64%	60-68	0.69	0.90	0.64-1.28	0.57
RFS	58%	49-69	57%	52-61	0.71	0.91	0.66-1.26	0.57
REL	31%	24-42	25%	21-29	0.29	0.90	0.59-1.38	0.63
NRM	10%	6-18	19%	15-22	0.091	1.45	0.79-2.66	0.23
II-IV aGVHD	29%	21-39	33%	29-37	0.42	1.15	0.77-1.71	0.49
III-IV aGVHD	4%	2-10	9%	7-12	0.096	2.34	0.84-6.52	0.10
Mod/sev cGVHD	15%	9-24	16%	13-19	0.85	1.24	0.70-2.19	0.47
All patients								
OS	63%	57-69	65%	63-67	0.97	0.87	0.69-1.09	0.22
RFS	56%	50-63	57%	55-59	0.81	0.86	0.70-1.06	0.15
REL	30%	24-36	26%	24-28	0.29	0.85	0.66-1.1	0.23
NRM	14%	10-20	17%	15-19	0.35	0.99	0.70-1.41	0.97
II-IV aGVHD	31%	25-37	30%	28-32	0.99	1.05	0.81-1.36	0.70
III-IV aGVHD	5%	3-9	7%	6-8	0.38	1.34	0.74-2.43	0.33
Mod/sev cGVHD	10%	7-15	12%	11-14	0.30	1.33	0.85-2.09	0.21

*HR from multivariable analyses, and bone marrow is the reference category; BM, bone marrow; PB, peripheral blood cells; 95%CI, 95% confidence interval; OS, overall survival; RFS, relapse-free survival; REL, relapse; NRM, non-relapse mortality; aGVHD, acute graft-versus-host disease; mod/sev cGVHD, moderate and severe chronic GVHD. Outcomes at 2 years, except for aGVHD – 6 months.



AUTOLOGOUS HSCT

FIFTEEN YEARS OF AUTOLOGOUS HEMATOPOIETIC CELL TRANSPLANTATION FOR LYMPHOMA IN BRAZIL

Abrahão Elias Hallack Neto[1]; Eliana Cristina Martins Miranda[2]; Antonio Vaz de Macedo[3]; Fernando Barroso Duarte[4]; Nelson Hamerschlak[5]; Carmem Bonfim[6]; Vanderson G. Rocha[7]; Mary E. Flowers[8]; Afonso Celso Vigorito[9].

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INTRODUCTION/OBJECTIVES:

Due to the lack of evidence regarding the best conditioning regimen for autologous hematopoietic cell transplantation (auto HCT) in patients with lymphoma, we analyzed the outcomes of patients in complete remission who received carmustine (BCNU) or lomustine (CCNU) as part of the conditioning regimens.

METHODS:

Adults patients with Hodgkin Lymphoma (HL), diffuse large B-cell lymphoma (DLBCL), and mantle cell lymphoma (MCL) transplanted between 2008 and 2023, reported to the Center for International Blood and Marrow Transplant Research (CIBMTR)/ Hematopoietic Cell Transplantation Brazilian Registry (HCTBR) were analyzed. The primary endpoints were disease free survival (DFS), overall survival (OS), non relapse mortality (NRM), and to evaluate the risk factors for survival rates in each disease group.

RESULTS:

669 patients were analyzed , 302 HL, 191 DLBCL, and 176 MCLThe 5-year DFS was 75% for HL, 57% for MCL and 44% for DLBCL. The OS was 85%, 64% and 54%, respectively. There was no difference in OS, DFS, and NRM among the different conditioning regimens in the lymphoma subtypes evaluated. In multivariate analysis, age at auto-HTC was associated with worse DFS in DLBCL (HR 1.02 95% CI 1.00-1.04 P= 0.01), male sex and age at auto HTC had worse DFS in MCL (HR 2.28 95% 1.06-4.91 P=0.03, HR 1.06 95% 1.01-1.10 P=0.006, respectively). Male sex and age had also worse OS in MCL (HR 2.31 95% 1.02-5.24 P=0.04, HR 1.04 95% 1.00-1.08 P=0.03, respectively).

CONCLUSION:

The present study has the largest number of patients among the Brazilian studies already published and can provide data on the safe use of lomustine in our population, with survival and NRM like those of carmustine regimens.

IMPACT OF GRAFT CRYOPRESERVATION STATUS ON ENGRAFTMENT SYNDROME IN AUTOLOGOUS SCT: DATA FROM A COHORT STUDY

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INTRODUCTION:

Engraftment syndrome (ES) is an early non-infectious complication of autologous stem cell transplantation (auto-SCT), associated with cytokine release. Since the diagnostic criteria were established, by Maiolino in 2001, the auto-SCT procedure has undergone significant updates, including the incorporation of plerixafor for mobilization, the use of non-cryopreserved grafts. Contemporary data on the incidence of ES remain scarce, particularly in light of these recent procedural and therapeutic changes.

OBJECTIVE:

To assess the frequency, risk factors, and clinical features of ES in patients undergoing auto-SCT at two centers in Rio de Janeiro, with an emphasis on recent procedural modifications. Additionally, we aimed to apply a cytokine release syndrome (CRS) severity score to grade ES cases.

METHODS:

This was a retrospective study involving 258 adult patients who underwent auto-SCT between 2020 and 2023 at two transplant centers (one public and one private) with differing access to novel therapies. Diagnoses included multiple myeloma/AL amyloidosis (61%), Hodgkin lymphoma (18%), and non-Hodgkin lymphoma (21%). ES was defined using Maiolino's criteria, and severity grading was performed according to the ASTCT 2019 consensus for CRS.

RESULTS:

ES was identified in 9% of cases (n = 23), occurring more frequently in female patients (13% vs. 5%; p = 0.034) and in those receiving non-cryopreserved grafts (37.5% vs. 7%; p < 0.001). Neither the use of plerixafor (8.7% vs. 9%; p = 0.954) nor the infused cell dose was associated with increased ES frequency. The public center showed a higher ES incidence compared to the private center (18% vs. 7%; p = 0.018), regardless of graft cryopreservation status. The most common clinical manifestations were fever (100%) and diarrhea (83%), while pulmonary infiltrates (13%) and skin rash (9%) were less frequent. All cases were classified as Grade 1 according to the ASTCT 2019 CRS grading system and received early corticosteroid therapy.

CONCLUSION:

ES remains a clinically relevant early complication of auto-SCT. Although easily identifiable, it entails significant morbidity and requires prompt management. According to the ASTCT 2019 grading system, all cases were mild. Notably, non-cryopreserved grafts were associated with a fivefold increase in ES frequency, highlighting the need for vigilance as fresh infusions become more common across transplant centers in Brazil.

POST-AUTOLOGOUS STEM CELL TRANSPLANT MAINTENANCE WITH BRENTUXIMAB VEDOTIN IN HIGH-RISK HODGKIN LYMPHOMA: A COMPARISON WITH STANDARD TREATMENT"

Aline Ferrari Martins[1]; Gustavo Zamperlini[1]; Jaqueline Takekoshi Teixeira[1]; Maria Alessandra Silva Salgado[1]; Julia Cervellini Yajima[1]; Cristiane Omae[1]; Mayra Troiani Franco[1]; Mariella Chequi Della Piazza[1]; Ana Beatriz Teodoro Borges[1]; Suelen Nascimento Meirelles[1]; Iva Loureiro Hoffmann[1]; Camila Maia Martin Daiggi[1]; Amilcar Cardoso de Azevedo[1]; Silvia Regina Brandalise Boldrini[1]

1. *Campinas - SP - Brasil*

INTRODUCTION

Classical Hodgkin lymphoma (cHL) is a rare pediatric malignancy, accounting for about 6% of childhood cancers. Although the overall survival rate is above 90% with standard treatment, relapsed cases remain challenging. These cases often require more intensive treatments, which carry a higher risk of toxicity. Brentuximab vedotin (BV), an anti-CD30 antibody-drug conjugate initially approved for adults, has demonstrated safety and efficacy in children. Combining BV with chemotherapy has shown a positive impact on event-free survival (EFS). Recently, the use of BV as post-ASCT maintenance therapy has emerged as a strategy to improve outcomes in high-risk patients.

OBJECTIVE

To evaluate whether post-ASCT maintenance therapy with BV improves OS and EFS rates compared to standard treatment in pediatric patients with high-risk cHL.

METHODS

Retrospective analysis of HL patients who underwent ASCR between 2005 and 2025. All patients received first-line, rescue, RT or maintenance treatment after ASCT at same hospital. ASCR were performed at different hospitals. No uniform treatment was used for first line or rescue chemotherapy. After 2020, patients with high-risk relapses (refractory disease, relapse within one year after therapy, bulky disease, or recurrence at sites of previous radiotherapy) received one year of brentuximab after ASCT. Almost all treated in the Public Health System.

Kaplan-Meier survival curves were used to estimate OS and EFS, and the log-rank test was applied for group comparisons.

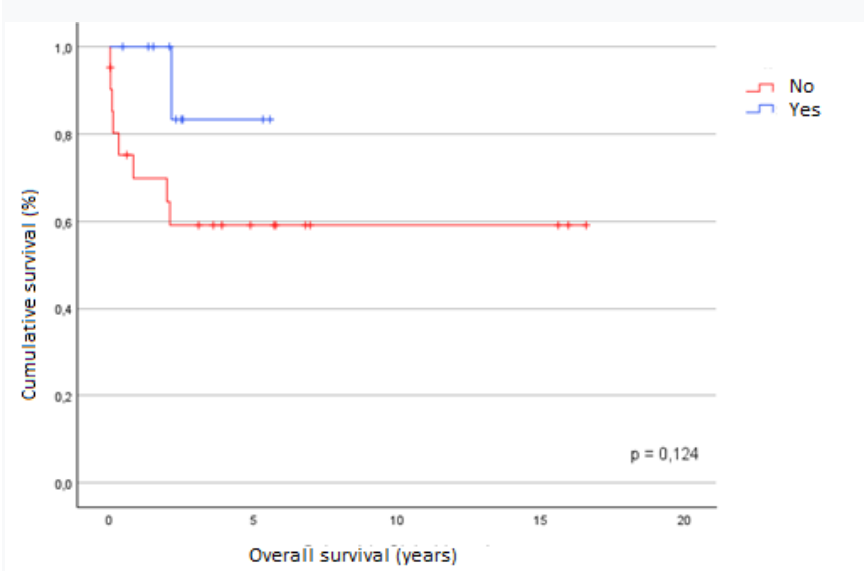
RESULTS

From 2005 to 2025, 331 children and adolescents were diagnosed with HL, of those 31 underwent ASCT and 28 (87%) were classified as cHL: 18 were male, 26 were older than 10 years, and 21 were classified as high-risk. The most used conditioning regimen was BEAM (58%); 21 patients were in remission prior to ASCT. Ten patients received BV as post-transplant maintenance. The 5-year OS was 83.3% ± 15.2% in the BV group versus 59.1% ± 11.2% in the non-BV group (p=0.12). The 5-year EFS was 74.1% ± 16.1% in the BV group versus 43.0% ± 11.4% in the control group (p=0.09). Patients who received additional post-ASCT therapy showed OS of 100% with radiotherapy (RT) alone, 50.0% ± 35.4% with RT+BV, 100% with BV alone, and 43.1% ± 13.2% with no complementary therapy (p=0.024). For EFS: 83.3% ± 15.2% with BV, 60.0% ± 21.9% with RT, 50.0% ± 35.4% with RT+BV, and 35.9% ± 12.8% without additional treatment.

CONCLUSION

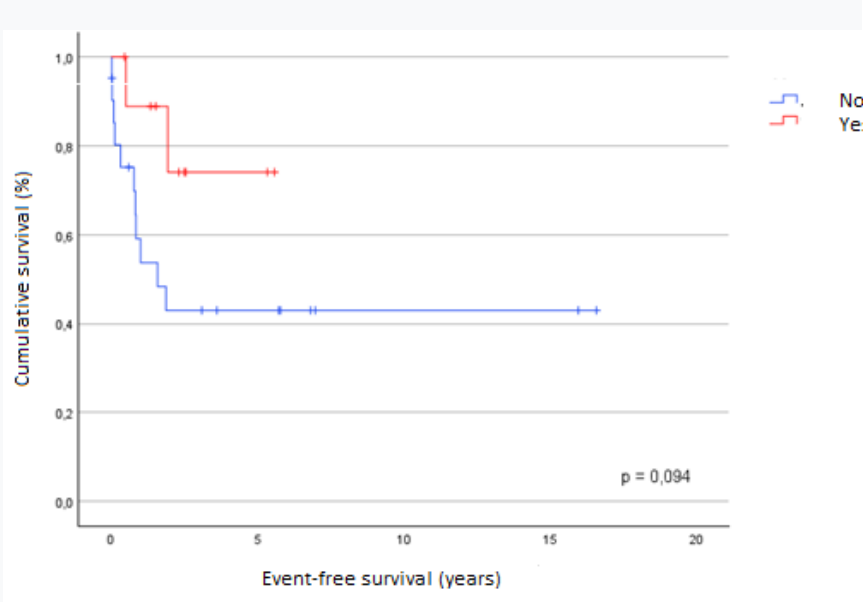
Post-ASCT maintenance therapy with BV in pediatric high-risk cHL patients showed a trend toward improved survival, though without statistical significance. The limited sample size may explain this. BV appears to be a viable option, with comparable or superior outcomes to radiotherapy and potentially fewer long-term adverse effects. Larger studies are needed to confirm these findings.

GRAFIC1. Overall 5-year survival of patients with Hodgkin's disease who underwent autologous HSCT, according to the use of Brentuximab therapy after transplantation



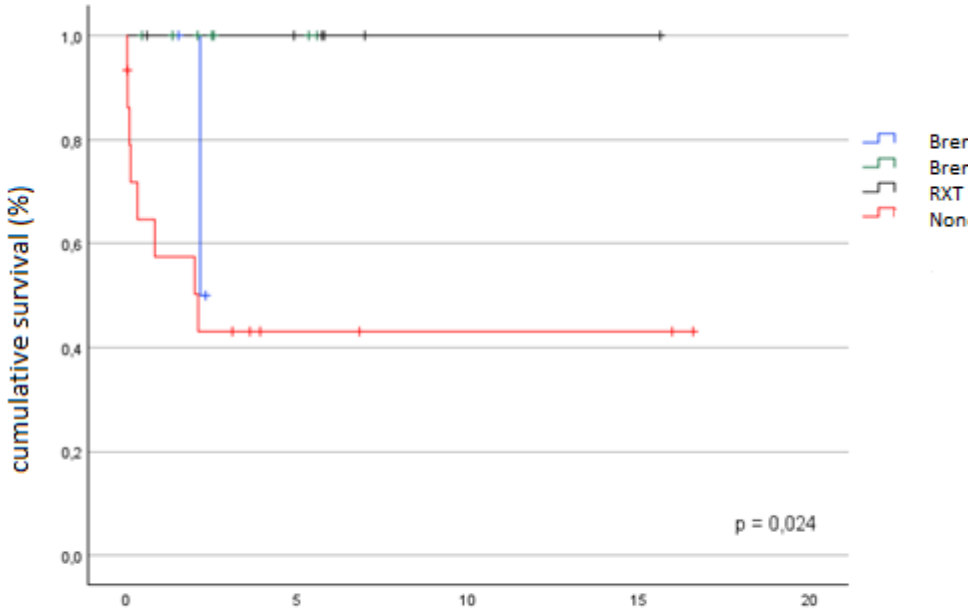
No SG = 59,1% ± 11,2% (N = 21 Live = 13)
Yes SG = 83,3% ± 15,2% (N = 10 Live = 9)

GRAFIC 2. Event-Free Survival at 5 years of patients with Hodgkin's Disease, undergoing autologous HSCT, according to the performance of Brentuximab therapy after transplantation



No SLE = 43,0% ± 11,4% (N = 21 RCC = 10)

GRAFIC3. Overall 5-year survival of patients at the Boldrini Children's Center with Hodgkin's Disease who underwent autologous HSCT, according to the type of complementary therapy performed after the transplant.



Brentuximab + RxT SG = 50,0% ± 35,4% (N = 3 Live = 2)
Brentuximab alone SG = 100% (N = 7 Live = 7)
RxT alone SG = 100% (N = 6 Live = 6)
None SG = 43,1% ± 13,2% (N = 15 Live = 7)

THE IMPORTANCE OF CYTARABINE IN CONDITIONING MANTLE CELL LYMPHOMA

Rafaella Cabral Samico[1]; Rony Schaffel[2]; Kelli Borges dos Santos[3]; Julia Diniz Ferreira[3]; Amanda Costa Moreira[3]; Abrahão Elias Hallack Neto[4].

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INTRODUCTION:

Mantle cell lymphoma (MCL) is a rare and heterogeneous lymphoproliferative neoplasm characterized by frequent relapses and poor long-term survival. Autologous hematopoietic cell transplantation (auto-HCT) has been used as a consolidation strategy in first remission, especially in younger patients. International studies suggest that the use of cytarabine in treatment can significantly improve clinical outcomes.

OBJECTIVE:

To evaluate the impact of the main factors on the survival of auto-HCT in patients with MCL, including the use of cytarabine in chemotherapy conditioning.

METHODS:

Longitudinal, retrospective, multicenter study with data obtained from medical records of patients diagnosed with MCL and undergoing autologous HSCT between 2004 and 2024. Patients from 2 centers in Minas Gerais and the Brazilian Registry of Mantle Cell Lymphoma were included. The variables were analyzed using descriptive statistics, Kaplan-Meier and Cox regression.

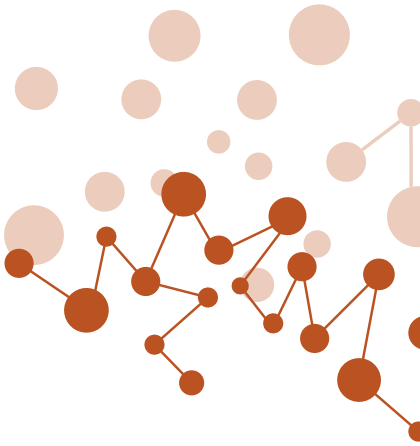
RESULTS:

Forty patients with mantle cell lymphoma undergoing autologous hematopoietic stem cell transplantation, aged between 40 and 70 years, 85% of whom were male, were included in the study. The majority (90%) had advanced clinical staging (III/IV) at diagnosis, evidencing the aggressiveness and late presentation pattern of the disease. The 3-year overall survival (OS) was 63.5% and the progression-free survival (PFS) was 59%. No statistically significant associations were observed with sex, age, staging and MIPI score. However, in the 3-year survival analysis, the use of cytarabine in the conditioning regimen demonstrated a positive impact on OS (64% vs. 27%; $p<0.01$) and PFS (75.3% vs. 27%; $p<0.01$). Multivariate Cox regression analysis reinforced this finding, with the use of cytarabine in conditioning being identified as an independent protective factor for mortality, reducing the risk of death by 89% (HR=0.11; 95% CI: 0.017–0.71; p=0.021).

CONCLUSION:

The use of cytarabine in the conditioning regimen was associated with better OS and PFS in this study, indicating its benefit also in the conditioning regimen for auto-HCT in patients with MCL. The findings reinforce the importance of its use in conditioning protocols for auto-HCT, corroborating what is already known about its benefits in induction chemotherapy for patients with MCL.

PEDIATRIC HSCT



ANTI-CD19 THERAPY (BLINATUMOMAB ®) AS A BRIDGE THERAPY FOR HEMATOPOIETIC STEM CELL TRANSPLANTATION IN BRAZILIAN PEDIATRIC PATIENTS WITH RELAPSED/REFRACTORY B-TYPE ACUTE LYMPHOBLASTIC LEUKEMIA PH-NEGATIVE

Talita Fernandes Nunes[1, 2], Roberia Mendonça de Pontes[2], Simone de Castro Resende Franco[3], José Carlos Martins Córdoba[2,3], Isis Maria Quezado Soares Magalhães[2,3], Agenor de Castro Moreira dos Santos Júnior[1,2], Ricardo Camargo[1,2] and Felipe Magalhães Furtado[2, 3, 4*]

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2. Laboratory of Translational Research, Children's Hospital of Brasilia, Brasilia, 70684-831 / Brazil
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INTRODUCTION:

B-Lineage Acute Lymphoblastic Leukemia (B-ALL) is the most common onco-hematological neoplasm in the pediatric population. Despite the efficacy of initial treatment, relapse or therapeutic resistance remains a challenge, with cure rates of less than 30% in early relapses and between 50% and 60% in late relapses. Blinatumomab (Blina®) is an immunotherapy that acts as a bispecific T-cell ligand, redirecting them to recognize and eliminate CD19+ leukemic cells.

OBJECTIVE:

Evaluate the outcomes of using Blina® in pediatric patients with Relapsed/Refractory (R/R) Ph-negative B-ALL treated at a public pediatric hospital in Brazil.

Methods: Retrospective, prospective and longitudinal cohort study that analyzed the use of Blina® as a bridge therapy for Hematopoietic Stem Cell Transplantation (HSCT) in 16 pediatric patients with Ph-negative B-ALL R/R, treated between 2017 and 2025. Data were obtained by reviewing electronic medical records. Children and adolescents (0-18 years) in first hematologic remission but with positive Minimal Residual Disease (MRD) ≥0.01% of blasts in the Bone Marrow (BM) after first-line treatment were included. The samples were analyzed according to EuroFlow protocols, with a minimum acquisition of 5×10^6 events/sample. A FACSCanto II cytometer and Infinicyt™ software were used. The study was approved by the ethics committee (44796221.9.0000.0144).

RESULTS:

In this study, 16 BM samples from pediatric patients were an alyzed, 62.5% male and 37.5% female, with a mean age of 6.81 years. Risk stratification was carried out according to the European Leukemia Net (ELN) criteria, with 1 patient being defined as low risk, 3 as intermediate risk, and 12 as high risk. Of the total, 12 patients underwent HSCT (10 haploidentical, 1 unrelated allogeneic and 1 with two transplants - haploidentical and related allogeneic). Samples were collected at different times during treatment with Blina®, including pre-transplant (n=16) and pre/post-transplant (n=3). MRD was considered negative when there were ≤0.01% blasts. Blina® was

administered by continuous intravenous infusion for four weeks: 5 mcg/m^2/24h for the first 7 days (1st cycle), followed by 15 mcg/m^2/24h (2nd cycle). A response to Blina® was observed in 81.25% (13/16) of patients, with 87.5% (n=14) using only the first cycle and 12.5% (n=2) both. The main adverse effect was fever; one patient had neurotoxicity, and treatment was interrupted. To date, 81.25% (n=13) have responded, some with complete remission. Among the non-responders, 18.75% (n=3) died from causes unrelated to the immunotherapy.

CONCLUSION:

The study showed a high response rate to Blina® in the first cycle, with a significant reduction in leukemic load and conversion to negative MRD, enabling early referral to HSCT. The results suggest substantial clinical benefits and logistical and economic advantages, with less need for multiple therapeutic cycles and prolonged hospitalizations.

KEYWORDS: Acute Lymphoblastic Leukemia, Immunotherapy and Pediatric Patients.

AUGMENTED CONDITIONING THERAPY MAY PREVENT SECONDARY GRAFT FAILURES IN PEDIATRIC HAPLOIDENTICAL HEMATOPOIETIC STEM CELL TRANSPLANTATION TO TREAT SICKLE CELL DISEASE (SCD)

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INTRODUCTION:

Most patients with SCD do not have an unaffected HLA-matched sibling donor (MSD), so alternative donor options are urgently needed. The Vanderbilt Global Learning Collaborative (VGC2) has led an international initiative to develop haploidentical HCT with post-HCT cyclophosphamide (PT-Cy) for SCD. Due to the 43% rejection rate with the original Hopkins protocol (Bolaños-Meade, 2012), thiotepa (TT) was added with excellent results in adults, but a 25% rejection rate in children (Kassim, 2024) despite a 2-month intensive marrow suppression with hydrea and exchange transfusions (HbS <30%, reticulocytes <10% and Hb 9-10g/dL). The

OBJECTIVE:

Compare the outcomes with the original VGC2 conditioning and the current augmented conditioning therapy in children, adolescents and young adults with SCD. Casuistic: 31 haploidentical HCT were performed in 4 institutions, 11 with standard-dose (Haplo) and 20 with augmented conditioning (AugHaplo).

METHODS:

Donors were ABO compatible, DSA negative and AA was preferred over SC trait. Conditioning included ATG 4.5mg/Kg, TT 10 mg/Kg, and fludarabine 150mg/m² in all patients. Haplo also had Cy 29mg/Kg and

TBI200cGy and AugHaplo, Cy 50mg/kg and TBI400 cGy in a single fraction with gonadal shielding. GVHD prophylaxis was PT-Cy, sirolimus and MMF in all patients.

RESULTS:

From Sep2016 to Feb2025, 11 Haplo and 20 AugHaplo were performed. Median age was 11 years, 50% female. Main indications were stroke or altered TCD/MoyaMoya; recurrent VOC and acute chest syndrome. Three AugHaplo were sucessfully desensitized for low DSA. Median CD34 marrow dose was 7.5x106/kg (2-11). Overall survival is 100% in Haplo and 95% in AugHaplo, with event free survival 94% in Haplo and 95% in AugHaplo with a median follow-up of 36 and 12 months, respectively. Secondary graft failure was documented in two Haplo on D+60 and D+180 and so far avoided by low dose DLI in 2 other Haplo with decreasing chimerism; all patients with AugHaplo, except one, have >95% chimerism, versus 7/11 Haplo. Grades III/IV acute and chronic GVHD were documented in 18% and 36% of patients with Haplo, compared with 10% and 20% with the AugHaplo. All patients had multiple viral reactivations (CMV, HHV6, BKV, EBV, respiratory infections). Other complications, in one patient each, were Guillan-Barre, PRES, and alveolar proteinosis secondary to sirolimus. One patient died of fulminant Rhinovirus infection after AugHaplo.

CONCLUSIONS:

All but one patient are alive. Increasing conditioning therapy has not increased the rates of neither acute nor chronic GVHD. Careful specific organ-toxicity evaluations and long term follow up will provide further data that can be used to inform patients about be risks/benefits of HCT. The planned next step is to maintain the treatment strategy while decreasing hypertransfusions to avoid red blood cell sensitization.

KEYWORDS: Sickle cell disease; haploidentical bone marrow transplant; secondary graft failure

FIGURE 1.

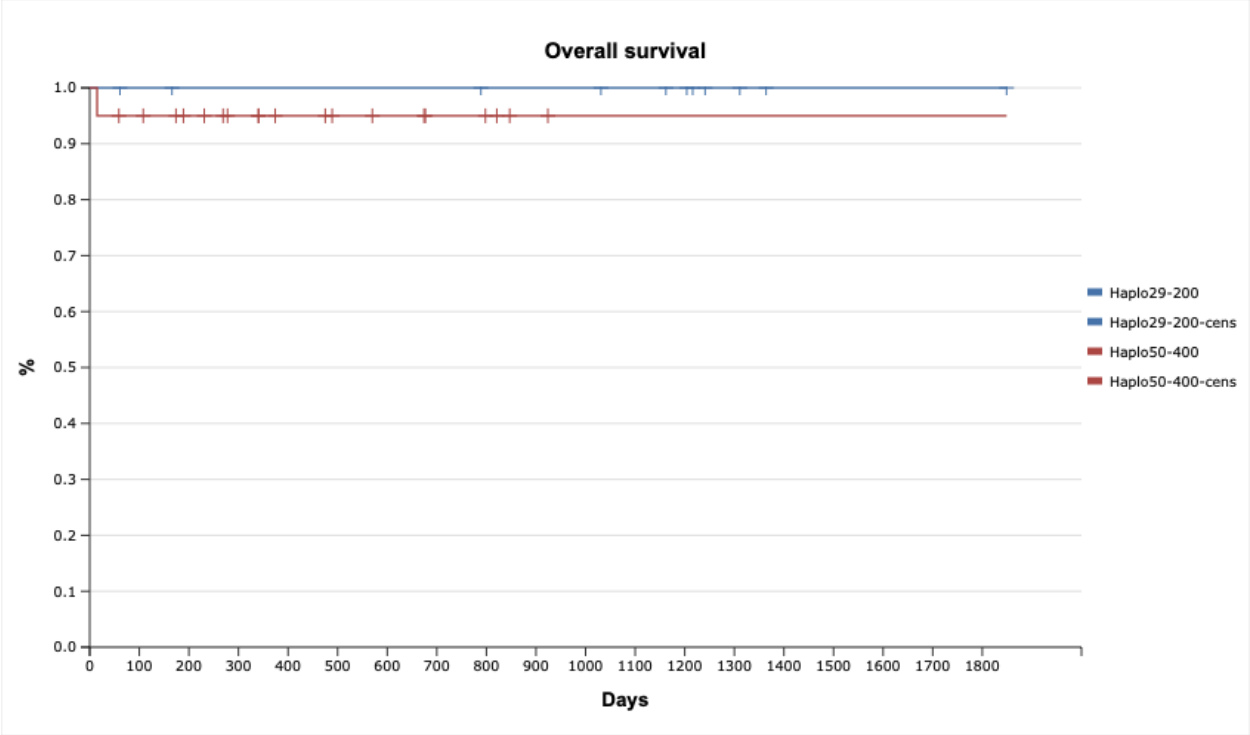


FIGURE 2.

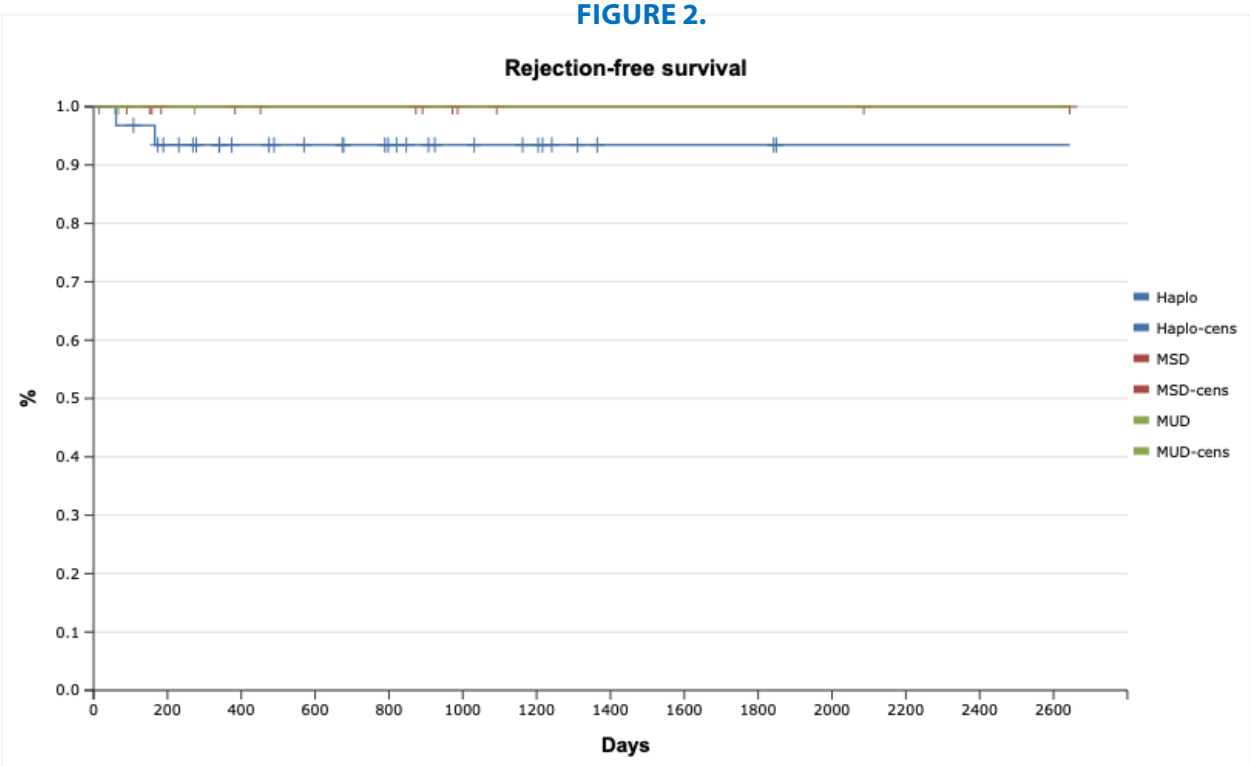
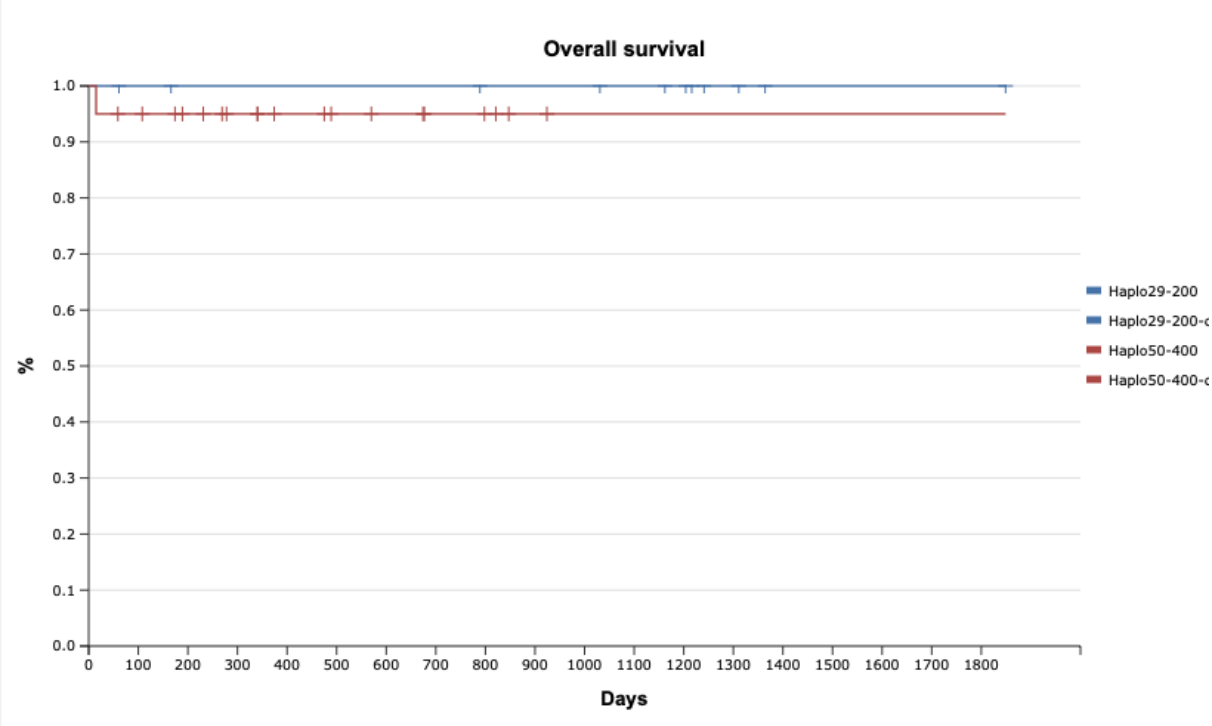


FIGURE 3.



ETOPOSIDE COMBINED WITH TOTAL BODY IRRADIATION DECREASES RELAPSE AFTER HAPLOIDENTICAL HEMATOPOIETIC STEM CELL TRANSPLANTATION TO TREAT PEDIATRIC ACUTE LYMPHOBLASTIC LEUKEMIA COMPARED WITH FLUDARABINE, WITHOUT INCREASING TRANSPLANT-RELATED MORTALITY

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INTRODUCTION:

Acute lymphoblastic leukemia (ALL) is the most common childhood cancer, accounting for 25% of the pediatric tumors. Advances in disease characterization and improved treatments have led to a current survival rate exceeding 85%. However, some children have refractory or relapsed diseases and their treatment involves reinduction with intensive chemotherapy +/- immunotherapy, followed by hematopoietic stem cell transplants (HCT) with total body irradiation (TBI). Haplo with PT-Cy for pediatric ALL was initiated based on the TBI-Fludarabine conditioning regimen used in adults by Solomon et al in 2012. However, TBI and Etoposide (TBI-VP) has been used for decades in pediatric HCT with unrelated (URD) or matched sibling donors (MSD). We have adopted this latter regimen also in Haplo with PT-Cy since it was suggested by the FORUM trial, but a direct comparison has neither been performed by our group nor has been presented or published yet, to the best of our knowledge. Objective: To compare overall survival (OS) and event-free survival (EFS) of consecutive pediatric patients with ALL undergoing Haplo TBI1200cGy with Fludarabine versus Etoposide

METHODS:

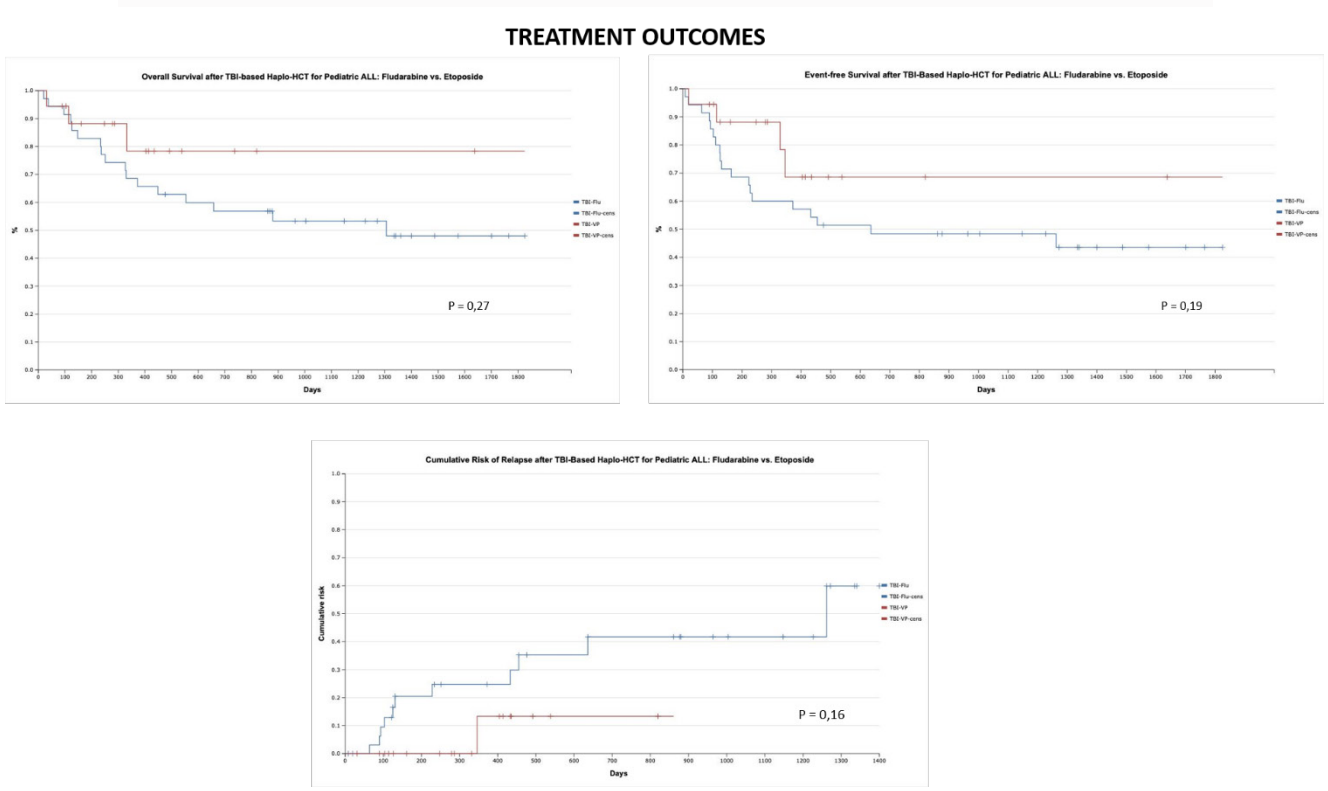
A retrospective analysis of all consecutive pediatric patients with ALL undergoing Haplo with TBI 1200cGy and Fludarabine (Flu-TBI - Solomon et al, 2019) or Etoposide (TBI-VP – Peters et al, 2020). Results: 53 Haplo for ALL with TBI 1200cGy were performed between February 2020 and February 2025 with a median follow-up of 2.4 years. All used standard

graft-versus-host disease (GvHD) prophylaxis with post-transplant Cyclophosphamide (PTCy). 87% of cases were B-cell ALL, 11% T-cell, and 2% mixed phenotype. 35/53 (66%) received Flu-TBI, and 18/53 (34%) TBI-VP. Engraftment was after a median of 15 days with fludarabine (13-21) and 17 days with VP (12-23). Peripheral blood grafts were used in 63% of the patients with Flu-TBI and in only one TBI-VP (11%). With Flu-TBI, 60% had acute GvHD (aGvHD) and 58% relapsed. Four patients underwent a second HCT, and all unfortunately died. With Flu-TBI, the EFS was 48% and OS 57% at 2.4 years. The 18 patients receiving TBI-VP, had 28% aGvHD, only one relapsed (extramedullary) and three died of TRM (16.6%). Therefore, after TBI-VP, the EFS was 69% and OS 78%. Compared with Flu-TBI, TBI-VP had a Hazard Ratio (HR) of relapse of 0.27 (95%CI 0.07-0.98), EFS of 2.02 (95%CI 0.8-4.9), OS of 1.94 (95% CI 0.7-5) although the small numbers do not allow to reach statistical significance.

CONCLUSION:

TBI-VP may have better outcomes compared with Flu-TBI and will continue to be used in our center when performing Haplo for ALL.

FIGURE 1.



SURVIVAL OUTCOMES FOLLOWING AUTOLOGOUS HEMATOPOIETIC CELL TRANSPLANTATION IN CHILDREN WITH NEUROBLASTOMA AT A PEDIATRIC ONCOLOGY CENTER

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INTRODUCTION:

Neuroblastoma (NB) is a pediatric tumor of the sympathetic nervous system, mainly affecting young children. High-risk NB (HR-NB) has a poor prognosis and limited response to conventional therapies, requiring intensive multimodal treatment. Autologous hematopoietic cell transplantation (auto-HCT), following high-dose chemotherapy, is a key strategy to eliminate residual disease. However, national data on its impact within Brazil's public healthcare system remain limited. Objectives: To describe the clinical and epidemiological characteristics of NB and evaluate outcomes of HR-NB patients undergoing auto-HCT.

METHODS:

A retrospective cohort study was conducted using electronic medical records of patients diagnosed with NB and treated at a public referral hospital in the Brazilian Midwest from 2018 to 2024. Statistical analyses included Kruskal-Wallis, Mann-Whitney U, and Chi-square tests. Overall survival (OS) and event-free survival (EFS) were estimated using the Kaplan-Meier method, with comparisons via log-rank test ($p < 0.05$). Follow-up was measured from diagnosis. Analyses were performed using SPSS. Ethics approval was obtained, and informed consent or assent (TCLE/TALE) was secured.

RESULTS:

Sixty-four patients were included; 58% were male, with a median age at diagnosis of 35 months (range: 1.3–165). Median time from symptom onset to diagnosis was 2 months (range: 0–18). The abdomen was the most frequent tumor site, particularly the adrenal gland (71%). Metastases were most common in bone marrow (62%) and bone (55%). HR-NB was diagnosed in 43 patients (67%). Of these, 29 underwent auto-HCT as part of salvage therapy, and 15 also received immunotherapy. For the entire cohort, five-year OS and EFS were 50% and 41%, respectively. Among HR-NB patients, five-year OS declined to 38% and EFS to 34%, indicating limited long-term disease control (Figure 1). Nonetheless, aggressive treatment strategies appeared to delay progression — the leading cause of death (31%, 20/64), particularly among children under 5 years of

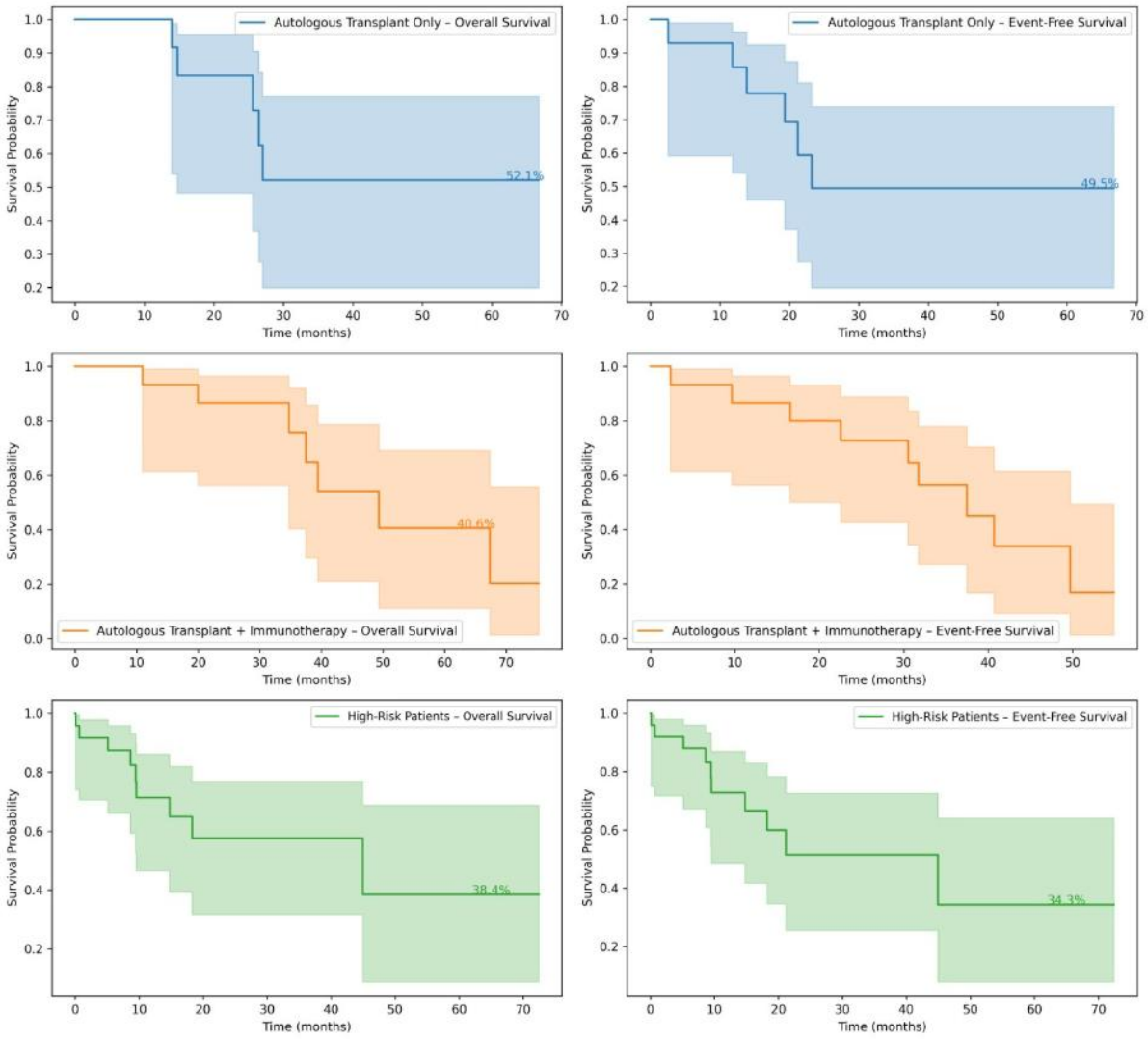
age with metastatic disease at diagnosis. Metastatic burden and N-MYC amplification were significantly associated with poorer prognosis (p = 0.034).

CONCLUSION:

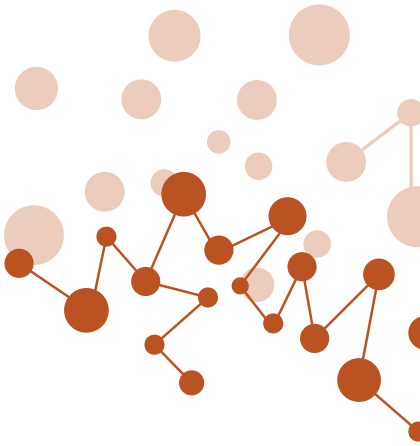
Delayed diagnosis and relapse remain major obstacles to improving NB outcomes. Despite these challenges, auto-HCT demonstrated potential in selected HR-NB cases, reinforcing the importance of expanding access to advanced therapies within Brazil's public healthcare system. As immunotherapy and targeted agents are increasingly incorporated into frontline regimens, defining the role of auto-HCT is essential to improve survival, balance toxicity, and guide personalized treatment strategies in this challenging pediatric malignancy.

KEYWORDS: Autologous hematopoietic cell transplantation, Neuroblastoma, Prognosis.

FIGURE 1.



INFECTIOUS COMPLICATIONS



BKV-ASSOCIATED HEMORRHAGIC CYSTITIS AFTER ALLOGENEIC HCT: PRELIMINARY DATA FROM A RETROSPECTIVE COHORT STUDY.

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INTRODUCTION:

Hemorrhagic cystitis (HC) is a frequent complication after hematopoietic cell transplantation (HCT). HC may occur until 72 hours after HCT secondary to conditioning toxicity, or later, often due to BK virus reactivation (BKV-HC). In the absence of effective and safe antivirals to control BKV-HC, supportive therapy (hydration, bladder irrigation and uroprotective drugs) has been mainly used. We retrospectively evaluated the clinical findings, outcomes and variables associated with BKV–HC.

OBJECTIVES:

Describe the cumulative incidence (IC), overall survival (OS) and non-relapse mortality (NRM) of BKV-HC.

METHODS:

Electronic medical records of allogeneic HCT recipients were reviewed from 2020 to 2024. Patients (pts) who performed more than one HCT were excluded. During this period, BKV DNAemia was monitored weekly by quantitative PCR (Mobius Life Science, Pinhais, Brazil). BKV-HC was defined according to ECIL 2018. The CI of BKV-HC, the variables associated with its occurrence, OS and NRM were determined by the software R.

RESULTS:

310 pts were included: 96 matched related donor (MRD, 31%), 145 haploidentical (Haplo, 46.8%) 57 matched unrelated donor (MUD, 18.4%) and 12 mismatched unrelated donor (mMUD, 3.4%). BKV (in blood and/or urine) was detected in 169 pts (54.5%). BKV-HC was confirmed in 27 pts (CI=8.7%). Eleven pts (40.7%) had hematuria grade II, 15 grade III (55.6%) and one grade IV (3.7%). BKV-CH CI was 25% in mMUD, 7% in MRD and 8% in haploidentical and MUD (p=0.23) (Figure 1). No patient received antiviral therapy. OS and NRM at 2 years were 66.8% and 20.6%, respectively. By multivariate analysis, variables affecting OS were non-myeloablative/reduced intensity conditioning (RIC), mMUD and haploidentical HCT, and performance status ≤80%. BKV-CH grade III-IV (HR 2.67, p=0.005) and RIC (HR 2.99, p<0.001) increased NRM, as shown below. We observed a low incidence of BKV-HC (8.7%) with no impact on OS. However, severe BKV-CH was frequent, and associated with increased NRM in this series (Figure 2).

KEYWORDS: Hemorrhagic Cystitis, BK Virus, Hematopoietic Stem Cell Transplantation.

FIGURE 1 – Cumulative incidence of BKV-HC in type of HSCT

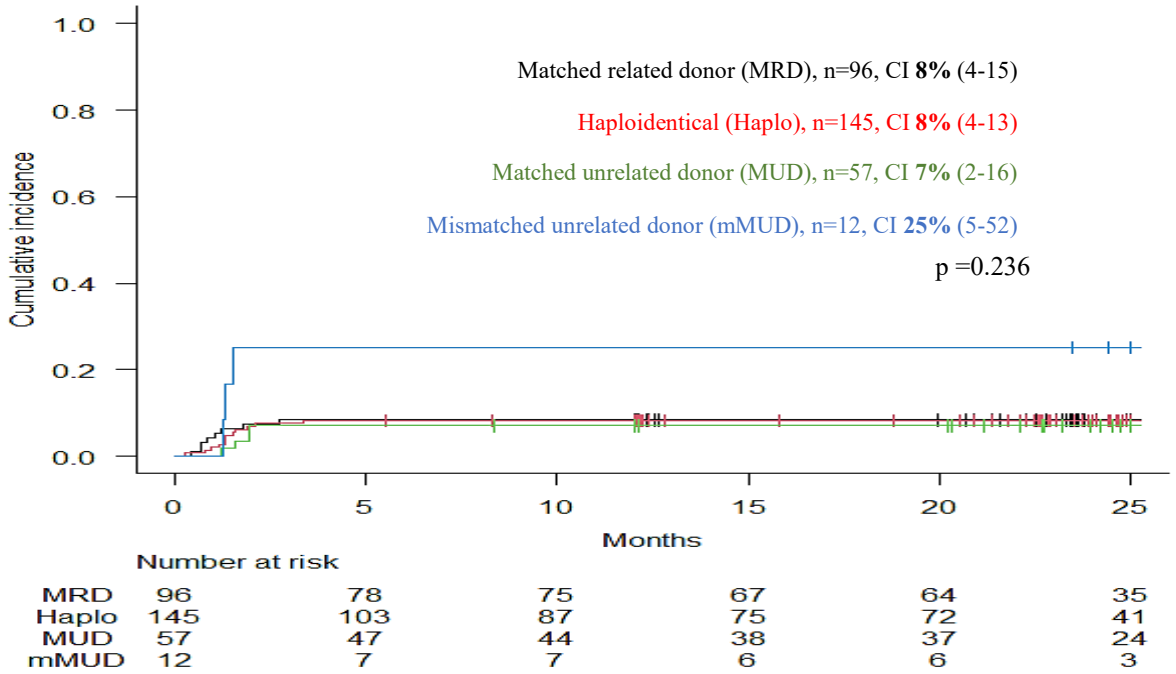
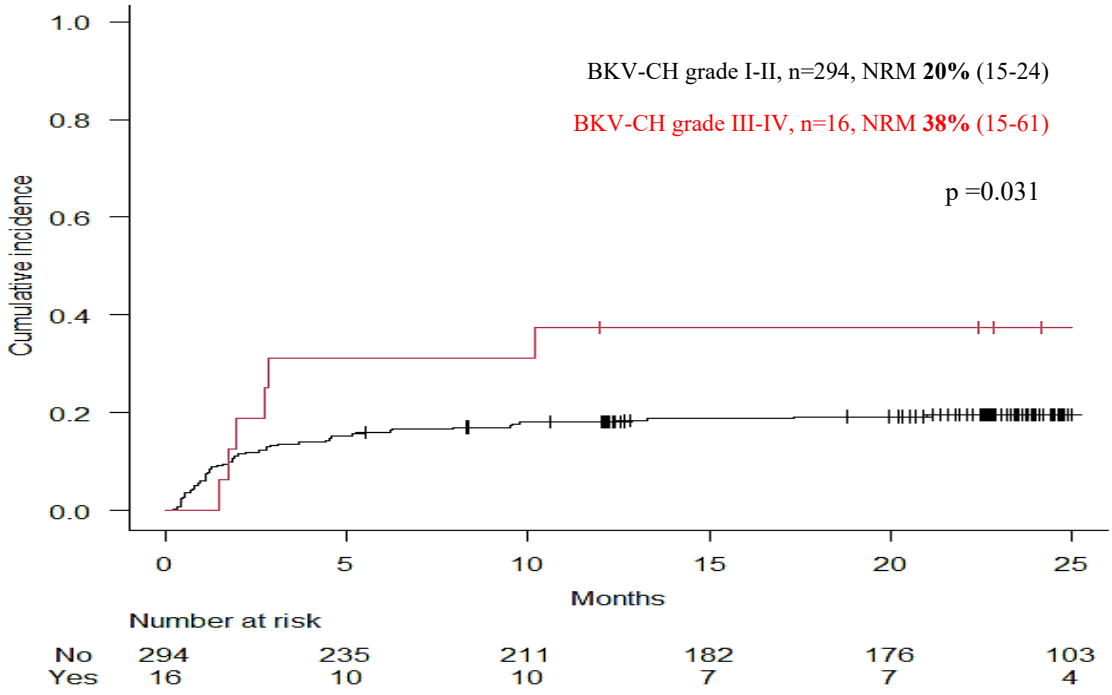


FIGURE 2 – Comparasion of cumulative incidence non-relapse mortality by BKV-CH grade



IMPROVING CYTOMEGALOVIRUS PREVENTION IN PEDIATRIC HEMATOPOIETIC STEM CELL TRANSPLANTATION: UPDATED OUTCOMES WITH LETERMOVIR

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INTRODUCTION:

Cytomegalovirus (CMV) is a major threat in pediatric allogeneic hematopoietic stem cell transplantation (HCT), especially in Brazil, where seroprevalence is high and matched donors are scarce. Ganciclovir, though widely used, has significant hematologic toxicity. Letermovir, a CMV-specific antiviral with a better safety profile, was approved by the FDA in August 2024 for children ≥6 months and ≥6 kg. Despite Brazil's high-risk epidemiologic context, the drug remains off-label for pediatric use locally. Objective: To present updated real-world data on letermovir prophylaxis in pediatric HCT recipients at a single Brazilian center.

METHODS:

Between December 2021 and June 2025, 44 patients under 21 years at high risk for CMV disease received letermovir as primary or secondary prophylaxis. High-risk was defined as recipient CMV seropositivity with donor seronegativity, or haploidentical/mismatched unrelated donor transplants (including isolated HLA-DP mismatches). Dosing was weight-based: <20 kg received 120–240 mg on alternate days, 21–30 kg received 240 mg daily, and >31 kg received 480 mg daily. HSV-seropositive patients also received acyclovir. CMV PCR was done weekly until day +100, and as needed thereafter. Interruptions for HHV-6 or HHV-7 treatment with ganciclovir were recorded.

RESULTS:

Median age was 6 years (1–21), and 18 (41%) were female. Most transplants were for malignant disease (66%) and haploidentical (70%). Letermovir was used as primary prophylaxis in 84% and secondary in 16%. Median start day was +5 for primary and +53 for secondary prophylaxis. Administration was mainly oral or via nasoenteral tube; the last 8 patients received IV letermovir until engraftment. Thirty patients (68%) had interruptions for HHV-6 or HHV-7, with 12 (27%) requiring multiple pauses. Two patients were empirically treated for suspected CMV disease (hepatitis and optic neuritis), although the etiology was not confirmed. One (2%) discontinued letermovir due to GI intolerance and later developed CMV esophagitis. Nine (20%) had low-level CMV viremia (80–620 IU/mL), all resolved without therapy changes. So far, 41 patients completed prophylaxis and 3 remained on letermovir. Median treatment duration was 110 days (16–612).

CONCLUSION:

Letermovir showed a very favorable safety profile and was effective in preventing clinically relevant CMV disease in high-risk pediatric HCT recipients. Despite frequent interruptions for HHV6 infections, most patients completed prophylaxis without complications. These findings reinforce the clinical value of letermovir in this vulnerable population and underscore the urgent need for regulatory approval and broader access in Brazil, where CMV burden is disproportionately high.

KEYWORDS: Cytomegalovirus, letermovir, pediatric

REFRACTORY CYTOMEGALOVIRUS (CMV) IN ALLOGENEIC HEMATOPOIETIC CELL TRANSPLANTATION (HCT): CLINICAL IMPACT AND RISK FACTORS

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INTRODUCTION:

CMV reactivation remains a burden in allogeneic HCT, especially in the challenging refractory scenario. CMV refractory (CMVref) is defined as the failure to reduce viral load by >1 log10 despite at least two weeks of effective antiviral therapy. It is associated with prolonged viremia, increased treatment duration, higher mortality, and a greater risk of CMV disease. Despite advances in prophylaxis and preemptive therapy, CMVref continues to challenge clinicians due to limited therapeutic options and worse outcomes.

OBJECTIVE:

This study aimed to characterize the frequency, clinical impact, and risk factors of CMVref in a cohort of HCT recipients, with a focus on viral dynamics, treatment outcomes, and associated mortality

CASUISTRY:

A retrospective observational study was conducted on 260 allogeneic HCT recipients between 2016 and 2023 at a tertiary care center in Brazil. Patients were monitored for CMV reactivation (CMVi) via quantitative PCR , and episodes were classified as clinically significant (CMVcs) and refractory (CMVref).

METHODS:

CMV surveillance was performed weekly until day +100 post-HCT and during immunosuppression. CMVi was defined as a positive PCR. CMVcs was defined as CMVi that required treatment, and CMVref was defined as <1 log10 reduction in viral load after ≥14 days of therapy. Clinical data, including viral load kinetics, treatment duration, GVHD, corticosteroid use, and outcomes, were analyzed. We excluded episodes with treatment shorter than 14 days. Statistical comparisons were made using chi-square, Mann-Whitney, and Kaplan-Meier tests.

RESULTS:

Out of 260 transplants involved in this study, CMVi occurred in 74%, with a total of 285 episodes. We observed a total of 160 CMVcs episodes, with 149 episodes with treatment longer than 14 days. Of those, 81 episodes (54%) were classified as CMVref.

CMVref episodes exhibited higher peak viral loads (median 6,083 vs. 1,334 IU/mL, p<0.001), longer viremia duration (median 78 vs. 36 days, p<0.001), and extended treatment (median 22 vs. 14 days, p<0.001) compared to non-refractory cases. Mortality during CMVref episodes was significantly higher (27% vs. 10%, p=0.012). Acute GVHD (57% vs. 45%, p=0.032) and high-dose corticosteroids (>1 mg/kg, 42% vs. 10%, p<0.001) were associated with CMVref. No difference was observed in donor type, conditioning intensity, or CMV serostatus between refractory and non-refractory cases.

CONCLUSION:

The majority of CMVcs episodes (54%) were defined as refractory CMV (CMVRef). These episodes were associated with prolonged viremia, extended treatment duration, and higher mortality compared to non-refractory cases. Key risk factors included acute GVHD and high-dose corticosteroid use."

KEYWORDS: Cytomegalovirus, refractory CMV, hematopoietic cell transplantation, viral load, GVHD, immunosuppression.

SAFETY AND EFFICACY OF TREATING PEDIATRIC LIFE-THREATENING VIRAL INFECTIONS IN CHILDREN AFTER ALLOGENEIC STEM CELL TRANSPLANTS

Carla Nolasco Monteiro Breviglieri[1], Camila Noronha Santos[1], Roseane Vasconcelos Gouveia[1,2], Valéria Cortez Ginani[1,2], Milena Reis Santos de Oliveira[1], Anna Beatriz Willemes Batalha[1], Gabriella Sayuri de Alencar[1], Márcia Puato Vieira Pupim[1], Juliana Francielle Marques[1], Joyce Ferreira Viana Dantas[1], Douglas Coutinho Ribeiro da Costa[1], Adriana Seber[1,2]

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INTRODUCTION:

Access to allogeneic hematopoietic stem cell transplantation (HCT) has grown in Brazil with the expansion of unrelated donor registries and the adoption of post-transplant cyclophosphamide (PT-Cy) for GVHD prophylaxis in mismatched transplants. However, both PT-Cy and HLA incompatibility delay immune reconstitution and increase the risk of viral reactivations. Infections—especially viral—are now the leading cause of death in this population. Despite being treatable, these infections are not covered by most healthcare plans. Essential antivirals like foscarnet and cidofovir/probenecid are unavailable commercially in Brazil due to lack of market interest. Without them, common viral infections such as HHV-6/7, BK virus, and adenovirus can become life-threatening. Although newer antivirals like letermovir and maribavir have been approved for CMV, pediatric indications are still lacking. ANVISA's Normative Instruction Nº352 (2025) permits importation, but the process is slow and not reimbursed—often requiring legal intervention. This delay poses a fatal risk to pediatric patients.

OBJECTIVE

Describe the safety and efficacy of foscarnet and cidofovir in this vulnerable setting and reinforce the need for guaranteed access to these life-saving therapies.

CASUISTIC:

Sixty patients underwent HCT from 2022 to 2024, 56 of them allogeneic. Eleven of them used Foscarnet (2 of them on two separate occasions), and 14, Cidofovir.

METHODS:

Antivirals were imported by the hospital for use in viral reactivations. Despite being listed as potentially necessary during HCT, each case requires individual insurance approval, which is routinely delayed and ultimately denied. Families are then forced to seek legal action or assume full financial responsibility. This process causes significant stress for patients, families, and healthcare teams.

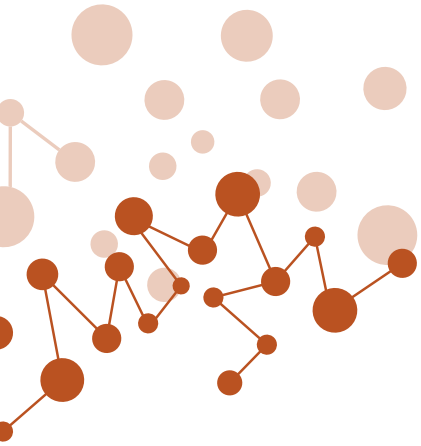
RESULTS:

Cidofovir was used to treat 3 disseminated adenoviral reactivations, 8 BKV infections and 3 refractory CMV or HHV6 reactivations. Foscarnet was administered to 11 children: 3 for CMV infections refractory to ganciclovir, and 8 for HHV-6 reactivations. Among those treated for HHV-6, 6 were treated with foscarnet after failed response to ganciclovir. Two patients received foscarnet due to ganciclovir-induced myelotoxicity, without prior use of ganciclovir. Two patients received foscarnet on two separate occasions: one for refractory CMV and later for HHV-6, and the other for HHV-6 reactivations—initially refractory to ganciclovir, and later without ganciclovir re-challenge due to prior resistance and concurrent myelotoxicity. All infections responded to therapy, with no serious adverse effects observed, but it would not have been the case without appropriate treatment.

CONCLUSION:

Cidofovir and foscarnet must be considered essential and readily available medications to be used in the HCT setting.

KEYWORDS: Cidofovir, foscarnet, equity



MULTIDISCIPLINARY

DEVELOPMENT OF A PSYCHOLOGICAL PROTOCOL FOR BONE MARROW TRANSPLANTATION (BMT) IN A FOUNDATION FOR THE ACCREDITATION OF CELLULAR THERAPY (FACT) - ACCREDITED INSTITUTION: AN EXPERIENCE REPORT

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INTRODUCTION:

Bone Marrow Transplantation (BMT) is a complex treatment that requires intensive care and a multidisciplinary approach. Psychology plays an essential role in this context, contributing through assessment, emotional support, and promotion of treatment adherence. In institutions accredited by the Foundation for the Accreditation of Cellular Therapy (FACT), such as ours, the standardization of care practices becomes indispensable. Developing a specific psychological care protocol is fundamental to ensuring quality, safety, and continuity of care. This work presents the experience of developing and implementing such a protocol in an accredited institution, contributing to best practices in patient care.

OBJECTIVE:

To describe the development of a psychological care protocol for patients eligible for BMT in an institution accredited by the FACT.

METHODOLOGY:

This is a descriptive study based on the professional experience between April 2024 and April 2025, in a private institution located in São Paulo, Brazil. The experience refers to the creation of a psychological protocol for adult and pediatric patients eligible for BMT. The institution's accreditation by the FACT, obtained in July 2024, contributed to the formalization and standardization of practices of care, including the work of the Psychology Service.

RESULTS:

The protocol was developed based on observed clinical demands and institutional routines, and it was implemented even before the institution received FACT accreditation. The initial psychological assessment takes place up to thirty days before transplantation and is conducted through a semi-structured interview. The assessment includes: medical history, identification of psychosocial risk and protective factors, transplant indication, psychiatric history, harmful habits, prior treatment adherence history, and understanding of the procedure and its complexity. The initial evaluation determines the indication, contraindication, or conditional indication for the procedure based on the patient's needs. Patients are monitored during hospitalization and reassessed thirty days after hospital discharge. During the analyzed period, 43 patients, both adults and children, were assessed and monitored according to the protocol's criteria.

CONCLUSION:

The development and implementation of the psychological protocol in the context of BMT aim to minimize risks, optimize outcomes, and improve the functional level and quality of life of the patients and their families. Systematized monitoring enables early identification of psychological risk factors that may compromise treatment and facilitates more effective interventions, promoting better treatment adherence. Standardizing practices enhances the quality of care provided and aligns the Psychology Service's performance with the principles of the FACT, contributing to the safety, comprehensiveness, and continuity of the treatment process for both adults and children.

DEVELOPMENT OF MACHINE LEARNING MODELS FOR PREDICTING EARLY DISCHARGE WITHOUT RISK OF EARLY READMISSION IN AUTOLOGOUS HEMATOPOIETIC CELL TRANSPLANTATION

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INTRODUCTION:

In Brazil, the waiting time for hematopoietic cell transplantation (HCT) is significantly affected by logistical and social constraints, and by the number of beds in a hospital ward. A predictive model capable of identifying patients eligible for early discharge prior to neutrophil engraftment — without an associated risk of early readmission—could help optimize inpatient bed use and reduce the risk of hospital-acquired infections.

OBJECTIVE:

This study aims to develop a machine learning (ML)-based predictive model to identify patients eligible for early discharge.

METHODS:

The study population included 387 patients over 18 years of age who underwent autologous HCT at a tertiary hospital between October 2018 and May 2025. Patients were classified eligible (value = 1) or not (value = 0) for early discharge. For this study, eligibility was defined as hospital discharge prior to neutrophil engraftment without readmission within 10 days. Three approaches were used for model training: (1) the original dataset, (2) 1:1 Propensity Score Matching (PSM), and (3) over-sampling by generating synthetic early discharge cases with Generative Adversarial Networks (GAN) at a 1:1/2 ratio. For each approach, Pearson correlation was calculated in reference to the original dataset. Data were split into training (75%) and testing (25%) subsets. Three predictive algorithms were trained using the following features: disease, age, Charlson Comorbidity Index, HCT-CI score, colonization status, conditioning protocol, oral mucositis grade, diarrhea grade, body mass index, and the number of infused CD34+ cells. Default hyperparameter settings from the Scikit-learn package (v.1.6.1) were used for all models. Models were evaluated based on the area under the Precision-Recall curve (AUC-PR) and the F1 Score, both ranging from 0 to 1.

RESULTS:

Of the 387 patients, 40 received early discharge (prior to neutrophil engraftment). However, seven were readmitted within 10 days (median: 5 (3-8) days), resulting in 33 eligible early discharge patients. Correlation analyses demonstrated a strong association between the GAN-generated dataset and the original data (r = 0.88), and a moderate correlation for the PSM-adjusted dataset (r = 0.68).

Among the evaluated algorithms, the Random Forest (RF) model presented the best performance in all approaches used, such as the dataset generated by GAN (AUC-PR: 0.86; F1-score: 0.74), the data adjusted by PSM (AUC-PR: 0.75; F1-score: 0.74) and the original dataset (AUC-PR: 0.52; F1-score: 0.40) (Figure 1 and Table 1).

CONCLUSION:

This pilot study supports the feasibility of applying machine learning models to predict patients eligible for early discharge without risk of early readmission, particularly when enhanced by data balancing techniques. Future research will aim to validate these findings in a larger cohort and assess the performance of more complex algorithms, including neural networks, to further improve predictive accuracy.

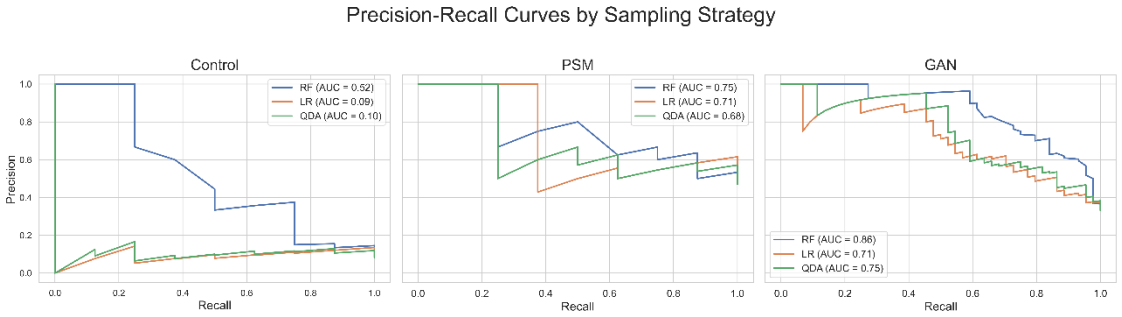
KEYWORDS:

Early discharge; Predictive Modeling; Hospital Resource Optimization.

TABLE 1. Models performance metrics.

Model	Control			PSM			GAN		
	Precision	Recall	F1-Score	Precision	Recall	F1-Score	Precision	Recall	F1-Score
RF	1	0.25	0.40	0.64	0.88	0.74	0.81	0.68	0.74
LR	0	0	0	0.50	0.62	0.56	0.67	0.55	0.60
QDA	0.12	0.12	0.12	0.58	0.88	0.70	0.58	0.64	0.61

FIGURE 1. AUC-PR for RF, LR, and QDA.



HOSPITAL SCHOOLING FOR PEDIATRIC PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION: PROMOTING EDUCATIONAL CONTINUITY AS PART OF THE COMPREHENSIVE CARE.

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INTRODUCTION:

Hematopoietic Stem Cell Transplantation (HSCT) in pediatric patients entails prolonged hospital stays, social isolation, and substantial disruptions in the educational trajectories. Within these circumstances, ensuring educational continuity represents a critical challenge. Although clinical and psychosocial aspects of HSCT have been extensively explored, there remains a lack of research focusing on formal hospital schooling as an organized educational practice, particularly within Latin American healthcare and educational frameworks.

OBJECTIVES:

To describe a successful structured hospital educational interventions, emphasizing curriculum adaptation, pedagogical strategies, the organizational structure and provision of educational services for pediatric HSCT patients.

METHODS:

This quantitative-descriptive study was conducted at a reference center and analyzed data from 131 students/patients who accessed educational services between 2022 and 2024. These patients were in reversal isolation precautions, thus, educational services were delivered individually, both during hospitalization and outpatient setting. Data were collected through the teachers' participation in multidisciplinary team weekly meetings, and systematic records were maintained within the institutional database.

RESULTS:

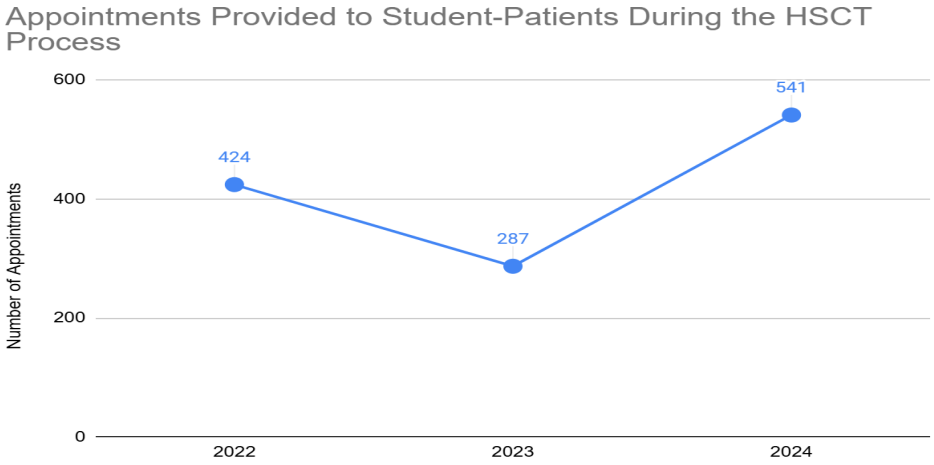
A total of 1252 educational interventions were recorded, 424 in 2022, 287 in 2023, and 541 in 2024, involving 131 students/patients (71.8% elementary and middle school, 16.8% high school, 8.4% non-enrolled youth, 3.05% others) supported by two hospital teachers specializing in mathematics and portuguese language. There was only one school failure. Among the 16.8% of high school students, 45.45% took the entrance exam for technical schools or universities at the hospital, of these, 9.09% were accepted. The pedagogical strategies aimed for continuity of learning, with curricular adaptations aligned to the students' clinical and cognitive conditions, prioritizing core competencies and

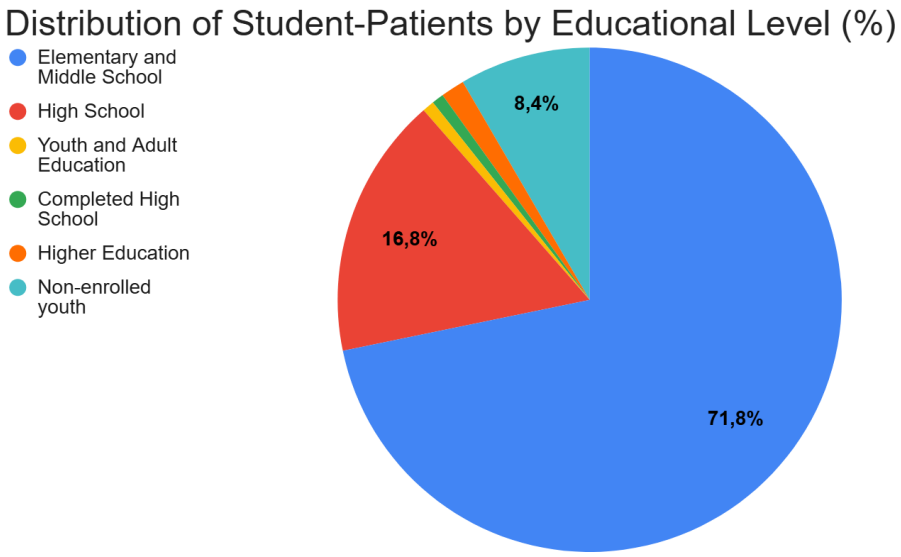
flexible learning objectives based on hospitalization duration and health status. These findings emphasize the importance of systematic record-keeping and continuous monitoring as essential strategies for effective management and pedagogical quality improvement.

CONCLUSION:

This study reinforces hospital schooling as an indispensable component of comprehensive pediatric oncological care, safeguarding the right to education and fostering sustained educational engagement despite severe illnesses. The promotion of hospital-based schooling necessitates the development of specific protocols tailored to student/patients needs, while the consolidation of institutional records enhances the visibility and value of such services, informing future research and the refinement of educational and healthcare policies.

KEYWORDS: Hospital Educational Care; Hematopoietic Stem Cell Transplantation (HSCT); Health Education; Multidisciplinary Team.





MULTIPLE MYELOMA AND POST-AUTOLOGOUS HSCT QUALITY OF LIFE: FUNCTIONAL, EMOTIONAL, AND SPIRITUAL FACTORS IN OLDER ADULTS

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INTRODUCTION:

Facing an onco-hematological disease in old age, especially after Autologous Hematopoietic Stem Cell Transplantation (HSCT), represents an additional challenge in a stage of life already marked by losses and limitations. Objective: To understand the factors that influence the quality of life (QoL) of elderly patients after autologous HSCT, considering functional, emotional, symptomatic, existential, and sociodemographic aspects. Sample: 28 patients diagnosed with multiple myeloma, undergoing autologous HSCT, with a mean age of 63.9 years (±8.4), mostly retired and male (57%).

METHOD:

The PHQ-9, Karnofsky Performance Status, EORTC QLQ-C30, and MLQ-QSV scales were administered and scored according to the instruments' guidelines during outpatient follow-ups. Data were analyzed using Spearman correlation and the Mann-Whitney U test ($p<0.05$).

RESULTS:

Functional domains showed high average scores: Physical Function (68.6 ± 22.7), Role Function (78.0 ± 28.0), Emotional Function (66.1 ± 34.0), Cognitive Function (76.2 ± 28.5), and Social Function (69.6 ± 33.4). The most prevalent symptoms were fatigue (36.5 ± 29.0) and pain (36.3 ± 32.7), followed by insomnia (32.1 ± 39.0). The mean PHQ-9 score was 7.9 (±5.46), suggesting mild to moderate depressive symptoms. The Karnofsky Index had a mean of 81.79 (±7.58). Global QoL averaged 72.3 (±25.6). Depression (PHQ-9) was negatively correlated with physical (rs = -0.63), emotional (rs = -0.48), cognitive (rs = -0.68), role (rs = -0.41) domains, and global QoL (rs = -0.48). Symptoms such as insomnia (rs = 0.59), dyspnea (rs = 0.49), and fatigue (rs = 0.41) were positively correlated with emotional distress. The Karnofsky Index was positively correlated with physical function (rs = 0.46) and negatively correlated with depressive symptoms (rs = -0.56). Lower income was associated with higher symptom burden and financial difficulties (nausea/vomiting: rs = -0.59; diarrhea: rs = -0.50; insomnia: rs = -0.43; financial problems: rs = -0.41). Religious practitioners had better cognitive function ($p<0.05$), less insomnia ($p=0.0048$), and higher overall functionality ($p=0.0316$).

CONCLUSIONS:

The quality of life of elderly individuals after HSCT is multifactorial and shaped by emotional, physical, and contextual elements. Depressive symptoms were associated with decreased functionality and global well-being. The interdependence of functional domains suggests that preserving physical and emotional capacity supports social engagement and autonomy. Spirituality emerged as a relevant protective factor, associated with better cognitive indicators and reduced insomnia. Lower income highlighted an additional vulnerability, linked to greater symptom burden and financial hardship. Investments in early screening and management of depressive symptoms, as well as functional and spiritual support, are essential to promote the comprehensive well-being of these patients.

KEYWORDS: quality of life, spirituality, autologous HSCT.

NUTRITIONAL PROFILE AND CORRELATION OF NUTRITIONAL STATUS OF ELDERLY INDIVIDUALS UNDERGOING AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a potentially curative treatment that can provide increased survival in patients diagnosed with hematologic neoplasms. The increase in population aging and the number of elderly individuals diagnosed with oncohematologic diseases is directly proportional to the trend of increasing the number of elderly individuals undergoing transplantation. During this phase of life, weight gain is common due to increased body fat and loss of muscle mass. Maintaining the nutritional status of this population is extremely important for a good clinical outcome. Objective: To describe the nutritional profile of elderly individuals who underwent autologous HSCT and to analyze the correlation of nutritional measurements.

METHODS:

This observational analytical study included elderly patients over 60 years of age who underwent autologous HSCT. Assessments were performed in the pre-transplant period; nutritional status was determined by mid arm circumference (MAC) and calf circumference (CC) in centimeters, by the Patient-Generated Subjective Global Nutritional Assessment (PG-SGA), and by Body Mass Index (BMI) in kilograms per square meter (kg/m^2). Data were expressed as means and proportions; the normality test used was the Shapiro-Wilk test; correlational analysis was calculated by Pearson's coefficient, with a significance level of $p<0.05$.

RESULTS:

29 elderly individuals were evaluated, with a mean age of 65.59 ± 3.97 years. 100% were diagnosed with multiple myeloma and 68.9% were male. The ASG-PPP showed that 93.1% of the patients were well nourished and 82.8% were eutrophic, according to MAC and CC, 88.9% had adequate muscle mass reserve. 34.5% of the patients were overweight and 34.5% were obese according to the BMI. The figure (A and B) demonstrates a positive correlation between BMI-AC, Pearson's R of |0.65| (p=0.0001 | 95% CI 0.38-0.82) and between BMI-CC, Pearson's R of |0.48| (p=0.007 | 95% CI 0.14-0.72). During hospitalization, 93.1% of the patients had an indication for oral nutritional therapy. The mean length of hospital stay was 17.76 ± 3.91 days, during which time, the mean percentage of weight loss was 4.09%. Conclusion: Considering the findings, a large part of the sample presented good or adequate nutritional status. A considerable proportion were overweight or obese. There was a correlation between BMI-MAC and BMI-PC, therefore, patients with higher BMI tend to have higher MAC and PC. This relationship reinforces the importance of using anthropometric measurements as auxiliary tools in nutritional risk screening. Furthermore, it makes clear the need for nutritional monitoring, aiming at the maintenance and recovery of nutritional status.

KEYWORDS: Hematopoietic Stem Cell Transplantation. Nutritional Assessment. Nutritional Status.

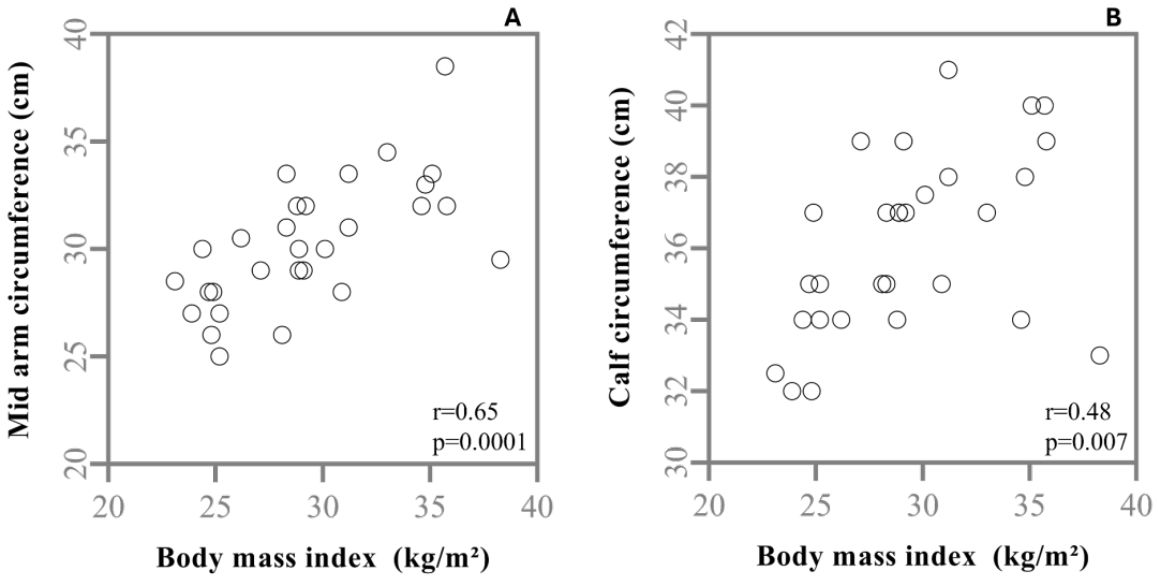

Figure 1: (A) Pearson correlation coefficient between BMI and MAC |0.65| demonstrating a strong and positive correlation with a statistically significant difference (p=0.0001). **(B)** Pearson correlation coefficient between BMI and CC |0.48| demonstrating a strong and positive correlation with a statistically significant difference (p=0.007).

PROTEOMIC PROFILING OF SALIVA IDENTIFIES METABOLIC AND INFLAMMATORY SIGNATURES LINKED TO ORAL MUCOSITIS SEVERITY IN ALLOGENEIC HEMATOPOIETIC CELL TRANSPLANTATION

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INTRODUCTION:

Oral mucositis (OM) is one of the most common and debilitating complications in patients undergoing allogeneic hematopoietic cell transplantation (allo-HCT). While proteomic analysis has been used to predict risk and prognosis in various pathophysiological contexts, its application to saliva samples in the investigation of OM remains limited.

OBJECTIVE:

To assess the abundance of salivary proteins in patients undergoing allo-HCT in relation to OM severity.

METHODS:

This prospective longitudinal study included patients undergoing their first allo-HCT. Saliva samples were collected immediately before conditioning (T0) and every two days until neutrophil engraftment. For analysis, T1 was defined as the sample collected on the day when the patient exhibited the highest OM grade. For patients who did not develop OM (grade 0, according to the WHO scale), a sample collected between days +7 and +10 post-transplant was used as T1. Participants were categorized into two groups: I) without OM (w-OM; grade 0) and II) with severe OM (s-OM; grades 3 and 4). Proteomic profiling was performed using mass spectrometry, and comparisons of group means at T0 and T1 were made using Student's t-test ($p \leq 0.05$). Functional enrichment of identified proteins was conducted using the enrichr-KG tool.

RESULTS:

Twenty patients were included. The mean age was 27.6 years (±9.64) in the w-OM group and 26.5 years (±11.41) in the s-OM group; most were male. At T0, enrichment analysis revealed a significant association with glycolysis in the s-OM group ($p=0.0001$), while the w-OM group showed a greater association with bacterial agglutination processes ($p=0.0036$), figures 1 and 2. At T1, the s-OM group exhibited strong associations with coagulation and complement cascade pathways ($p<0.0001$) and platelet degranulation ($p<0.0001$).

Conversely, the w-OM group was more associated with glycolysis ($p<0.0001$) and glucose catabolism to pyruvate ($p<0.0001$), figures 3 and 4. Thus, at T0, glucose metabolism pathways were predominant in the s-OM group, whereas antimicrobial activity was more prominent in the w-OM group. At T1, the s-OM group showed increased inflammatory and hemostatic responses, while the w-OM group displayed a delayed metabolic shift towards glucose utilization.

CONCLUSION:

This is the first study to identify salivary proteins and related biological processes associated with OM severity in allo-HCT patients. The w-OM group demonstrated a more adaptive and regenerative metabolic profile, suggesting preserved epithelial function and repair capacity. In contrast, the s-OM group exhibited upregulated inflammatory and coagulation pathways. These findings highlight potential biomarkers for risk stratification and therapeutic targeting in the management of OM during allo-HCT.

KEYWORDS:

Allogeneic Hematopoietic Cell Transplantation, Oral Mucositis, Proteomic Analysis

Figure 1. Functional enrichment of salivary proteins at T0 in patients with severe oral mucositis (grades 3–4), identified using Enrichr-KG. Green nodes show proteins (ALDH3A1, PGK1, FABP5, CAPG); colored nodes represent enriched biological processes (e.g., glycolysis, immune response, epithelial integrity).

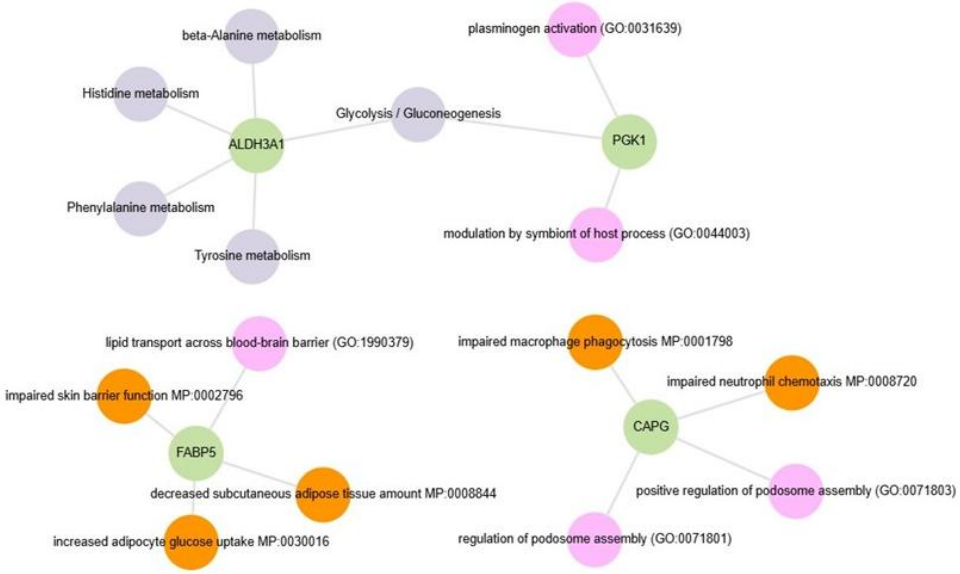


Figure 2. Functional enrichment of salivary proteins at T0 in patients without oral mucositis (grade 0), using Enrichr-KG. Green nodes represent proteins (e.g., FGB, BPIFB1); colored nodes indicate enriched processes such as fibrinolysis, bacterial agglutination, and innate immune response.

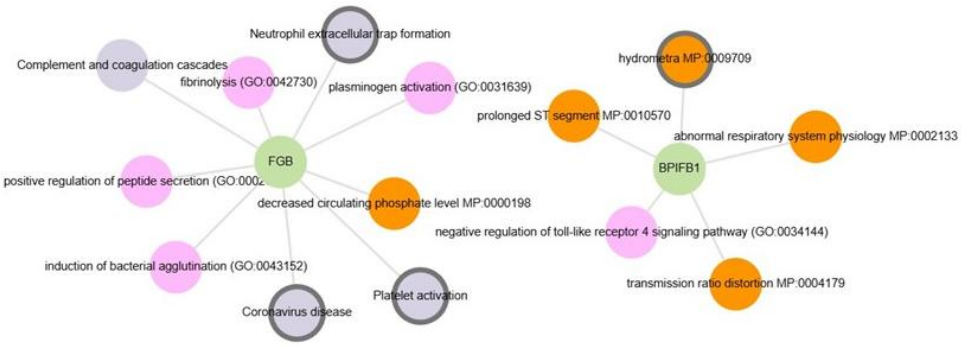


Figure 3. Functional enrichment of salivary proteins at T1 in patients with severe oral mucositis (grades 3–4), using Enrichr-KG. Green nodes (e.g., FGA, FGB, CFB, A2M, CFH) are linked to pathways including coagulation, platelet degranulation, complement activation, and impaired wound healing, reflecting intense inflammatory and hemostatic activity.

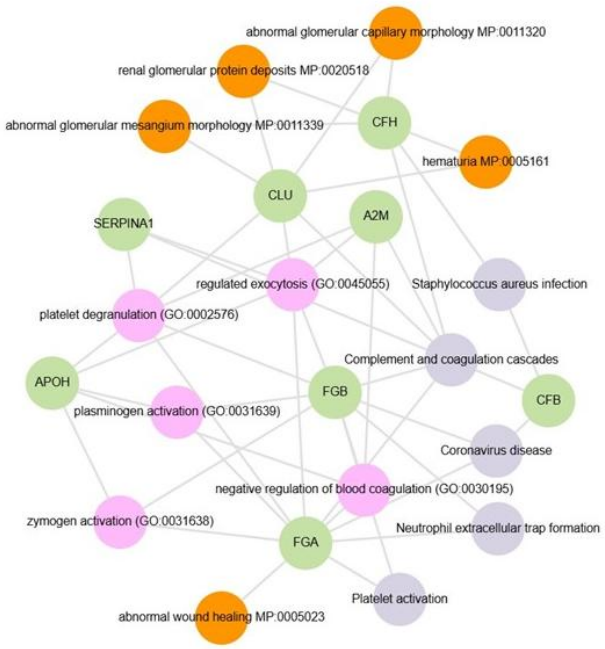
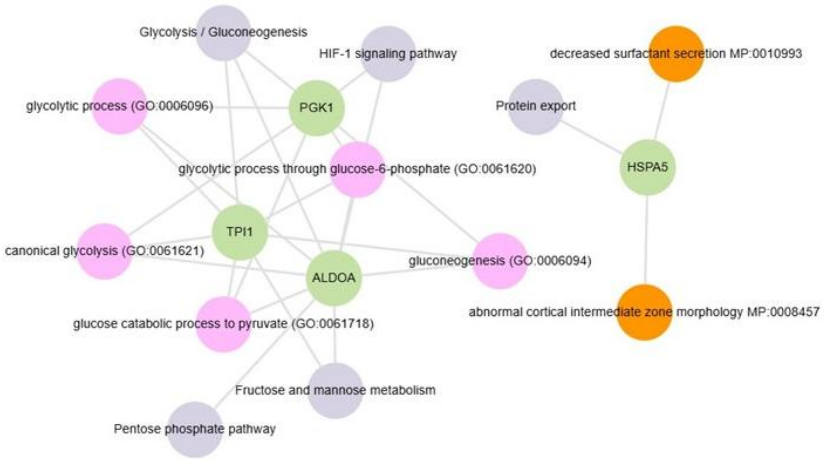


Figure 4. Functional enrichment of salivary proteins at T1 in patients without oral mucositis (grade 0), analyzed with Enrichr-KG. Green nodes (e.g., PGK1, TP1, ALDOA, HSPA5) are involved in glycolysis, gluconeogenesis, sugar metabolism, and protein stress response, indicating a protective metabolic profile without inflammatory markers.



REDESIGNING PATHS: STRATEGIES AND CHALLENGES IN THE IMPLEMENTATION OF A NAVIGATION PROGRAM IN ONCOHEMATOLOGY AND BONE MARROW TRANSPLANTATION

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INTRODUCTION:

Nurse-led navigation is recognized as a key differentiator in oncology services in Brazil, offering substantial benefits throughout the patient care continuum. In a high-complexity hospital setting, a navigation program focused on oncohematology and bone marrow transplantation (BMT) patients was implemented. This initiative was designed based on the identification of access barriers within the healthcare system and the comprehensive mapping of the patient journey, from diagnosis to discharge. The program's primary aim is to provide support, assist patients in coping with the emotional and clinical impact of their diagnosis, and mitigate factors that may delay the start of treatment.

OBJECTIVE:

To evaluate the implementation of a nurse navigation program for oncohematology patients, analyzing the associated challenges, initial outcomes, and future perspectives. The overarching goal was to foster continuous improvements in care delivery, ensuring a safer and more streamlined treatment experience.

METHOD:

This descriptive observational study documents the implementation of the navigation program at a high-complexity hospital. The model adopted was based on the guidelines developed by The GW Cancer Institute at George Washington University and adapted to the hospital's operational context. The program serves oncohematology patients indicated for treatment and external patients referred specifically for hematopoietic stem cell transplantation.

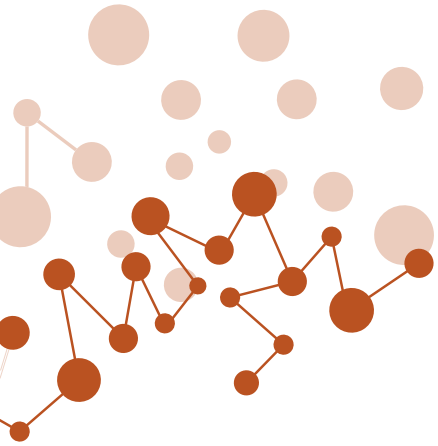
RESULTS:

The implementation yielded significant outcomes in three main areas: Reduced Time to Treatment Initiation – The average waiting time decreased from 25 to 10 business days following treatment decision-making. Remote Toxicity Management – This strategy proved highly effective, enabling home-based care in 97% of cases and significantly reducing the need for hospital admissions. Patient Onboarding Experience – Early patient engagement, starting from the first consultation stage, resulted in a Net Promoter Score (NPS) of 98%, reflecting exceptionally high levels of patient satisfaction with the support received.

CONCLUSION:

The navigation program has emerged as a strategic asset in enhancing care quality, ensuring a safer and more efficient patient journey by identifying and addressing access barriers. Future challenges include refining evaluation metrics and strengthening program monitoring to assess its long-term impact on patients' quality of life and the overall efficiency of the healthcare system.

KEYWORDS: Oncohematology, Patient Navigation, Bone Marrow Transplantation



GENERAL TOPICS

INCIDENCE, SEVERITY, AND ASSOCIATED FACTORS OF ACUTE GRAFT-VERSUS-HOST DISEASE: 21-YEAR SINGLE-CENTER ANALYSIS

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INTRODUCTION:

Acute Graft-versus-Host Disease (aGvHD) is an immune-mediated complication of allogeneic hematopoietic cell transplantation (HCT), triggered by donor T cells attacking recipient epithelial tissues, especially in the gastrointestinal tract, liver, and skin. Despite prophylactic immunosuppression, aGvHD remains frequent and clinically significant, particularly in grades II–IV, which require treatment and are associated with worse outcomes. Among affected patients, it is estimated that 35% to 50% develop corticosteroid-refractory disease, a condition linked to high morbidity and mortality. In this context, understanding the incidence, severity, and associated factors of aGvHD is essential not only to guide risk stratification and preventive strategies but also to help estimate the proportion of patients at risk for steroid-refractory disease, reinforcing the need for public health policies that ensure access to second-line therapies.

OBJECTIVE:

To evaluate the incidence, severity, and associated factors of aGvHD in HCT recipients.

METHODS:

Retrospective, single-center study including patients who underwent allo-HCT between 2002–2023. Acute GvHD was graded I–IV using clinical records. Variables collected included age, sex, race, disease, stem cell source (bone marrow [BM] or peripheral blood [PB]), donor type (MRD, MUD, MMUD, haploidentical), conditioning, and calcineurin inhibitor used (cyclosporine [CSA] or tacrolimus [TAC]). Bivariate analyses and univariate logistic regression were performed (SPSS v21).

RESULTS:

Among 634 allo-HCT recipients, 360 (60.1%) developed aGvHD: 66 (18.3%) grade I, 191 (53.1%) grade II, 65 (18.1%) grade III, and 38 (10.6%) grade IV. Incidence of grade II–IV and III–IV was 49.1% and 17.2%, respectively. Risk factors for grade II–IV included: adult age (≥18) (OR 1.41; 95% CI 1.00–1.99; p=0.049), PB source (OR 1.62; CI 1.14–2.30; p=0.007), MMUD (OR 2.56; CI 1.19–5.50; p=0.015), White race (OR 1.40; CI 1.01–1.94; p=0.040), and CSA use (OR 1.76; CI 1.04–2.97; p=0.033). White race was also associated with any-grade aGvHD (OR 1.76; p=0.001). Patients with grade II–IV were older (mean 31.93 vs.

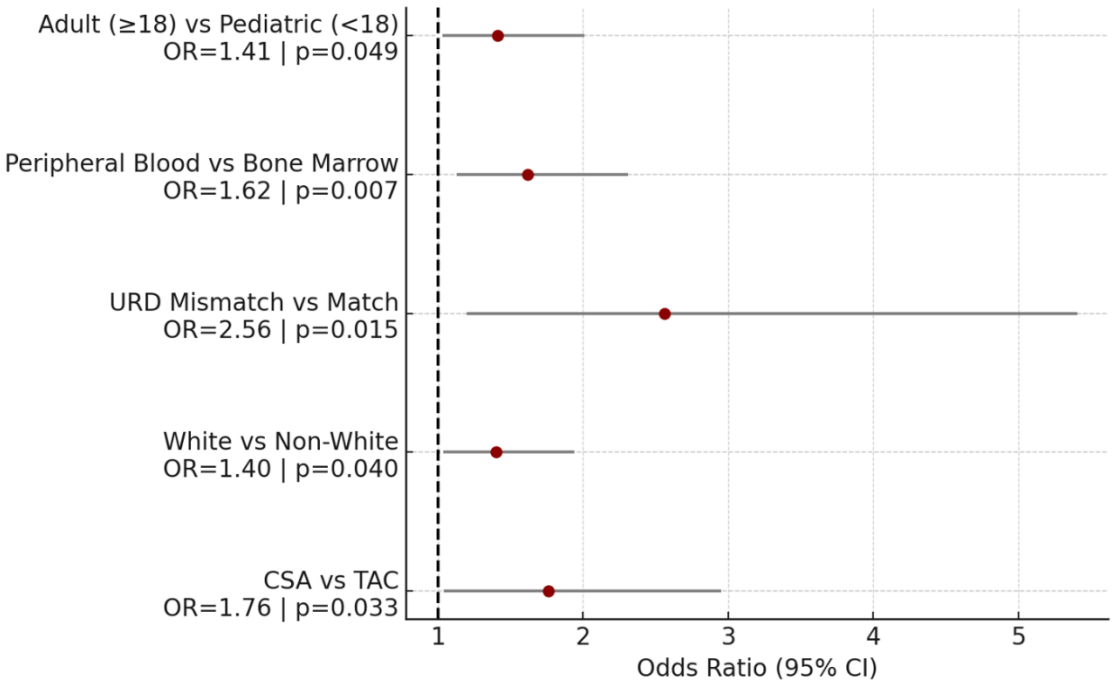
28.56 years; p=0.012), and those with grade III–IV received higher CSA doses in the first 21 days (191 vs. 174 mg/day; p=0.016).

CONCLUSION:

The high frequency of aGvHD, particularly grades II–IV, underscores its clinical relevance in the post-HCT setting. Adult age (≥18 years), PB as the stem cell source, MMUD, White race, and cyclosporine use for prophylaxis (compared to tacrolimus) were associated with an increased risk of acute GvHD II–IV. By characterizing the incidence, severity, and associated factors of aGvHD, this study offers valuable epidemiological data that can inform future investigations focused on treatment response and outcomes. These findings represent a necessary foundation for the development of prevention strategies and future public health planning, particularly in settings like the Brazilian SUS.

KEYWORDS: Acute Graft-Versus-Host Disease; Hematopoietic Cell Transplantation; Risk Factors

FIGURE 1. Univariate logistic Regression for acute GvHD Grades II-IV and associated factors.



LESSONS FROM THE HUMANIZED GVHD MODEL: SPINAL CORD AND DORSAL ROOT GANGLIA AS EMERGING TARGETS IN NEURO-IMMUNE CROSSTALK

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INTRODUCTION:

Graft-versus-host disease (GVHD) is a systemic inflammatory disorder that arises when donor immune cells recognize antigenic differences in the host following allogeneic hematopoietic stem cell transplantation (allo-HSCT), primarily due to disparities in major histocompatibility complex (MHC) antigens. While the intestine, liver, spleen, and skin are the most studied GVHD target organs, emerging evidence suggests that the nervous system may also be affected.

OBJECTIVES:

To characterize the cellular and molecular profile of the spinal cord and dorsal root ganglia (DRG) in mice subjected to GVHD.

METHODS:

C57BL/6 mice received 9 Gy of gamma irradiation, followed the next day by intravenous infusion of 3×10^7 splenocytes and 1×10^7 bone marrow cells from BALB/c donors. GVHD mice were monitored for survival and clinical signs. The spinal cord, thoracic and lumbar DRG, and respective meninges were collected at peak disease severity (score 10–12). Cytokine and chemokine expression was assessed by polymerase chain reaction (PCR), and inflammatory cell infiltration by immunofluorescence. To validate DRG/meninges as inflammatory targets, a humanized GVHD model (GVHDhu) was used by transplanting 1×10^7 human peripheral blood mononuclear cells (PBMCs) into NOD-scid IL2Rγ-null (NSG) mice. Flow cytometry was performed to detect human CD45+ leukocytes in blood and DRG/meninges. Student's t-test was used for two-group comparisons; Mann-Whitney and Kruskal-Wallis tests were applied for nonparametric data, with Dunnet post-test. Results were expressed as mean ± SEM. $P < 0.05$ was considered significant.

RESULTS:

GVHD mice showed progressive clinical deterioration compared to controls. PCR showed increased mRNA expression of IL-1β and TNF-α in the spinal cord, and IL-1β, CCL2, and IFN-γ in the DRG. Immunofluorescence revealed leukocyte infiltration in DRG. Flow cytometry confirmed human CD45+ cells in DRG/meninges, supporting a local inflammatory response. Behavioral tests showed reduced locomotion and rearing in GVHD mice, indicating functional impact. Conclusion: GVHD induces neuroinflammation in the spinal cord and DRG, identifying them as novel target organs. Future steps include phenotypic profiling of infiltrating leukocytes and investigation of GVHD-induced pain-related behaviors. Ethical approval: Protocol 10/2024.

KEYWORDS: DRG; Humanized GVHD model; Allo-HSCT.

OUTCOME ANALYSIS IN PATIENTS WITH ACUTE MYELOID LEUKEMIA UNDERGOING ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION BASED ON PRE-TRANSPLANT EVALUATION OF MEASURABLE RESIDUAL DISEASE

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1- A Beneficência Portuguesa de São Paulo
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INTRODUCTION:

Acute Myeloid Leukemia (AML) is a clonal hematopoietic malignancy characterized by bone marrow failure due to leukemic infiltration. It is the most common acute leukemia in adults, with a median age at diagnosis of 68 years. Classification and risk stratification rely on cytogenetic and molecular abnormalities, which are critical for guiding therapy. Remission rates decline with age and response assessment is based on morphological criteria and Measurable Residual Disease (MRD) analysis. Persistent MRD correlates with increased relapse and decreased survival. Allogeneic Hematopoietic Stem Cell Transplantation (HSCT) remains a key curative strategy, especially in intermediate- and high-risk AML, though the optimal role of MRD in guiding HSCT approaches, including choice of conditioning regimen (MAC vs. RIC), remains under discussion.

OBJECTIVES:

To evaluate cure and relapse rates in AML patients undergoing allogeneic HSCT based on pre-transplant response status following induction therapy.

CASUISTIC:

This study included 64 AML patients who underwent allogeneic HSCT between 2019 and 2024 at a single center.

METHODS:

Retrospective study of adult AML patients who received induction therapy followed by allogeneic HSCT. Pre-transplant response was classified as MRD-negative, MRD-positive, or active disease. Statistical analysis included chi-square and Student's t-tests, with relapse and mortality estimated using competing risk methods.

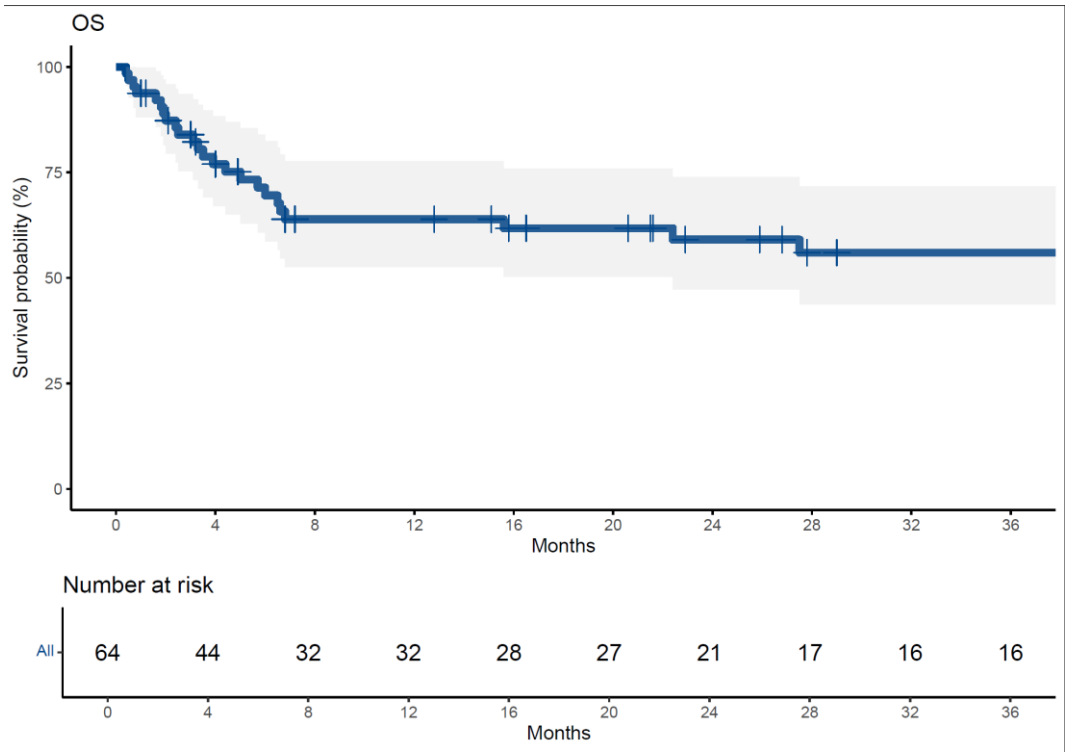
RESULTS:

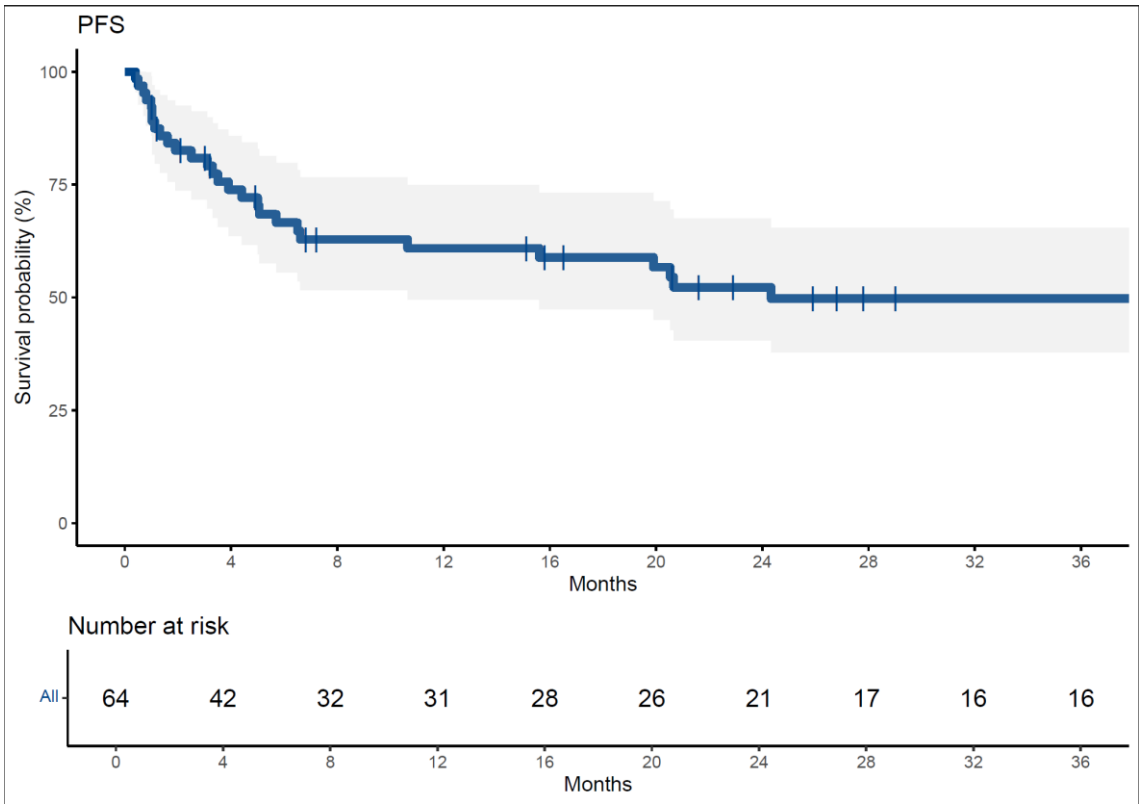
The median age was 48.5 years [19–83]. Stem cell source: peripheral blood in 93.8%, bone marrow in 4.7%, and cord blood in 1.6%. Conditioning regimen was RIC in 89.1% and MAC in 10.9%. Donor types included haploidentical (59.3%), matched related (23.4%), matched unrelated (6.2%), and mismatched unrelated (9.3%). ELN 2022 risk classification: high risk in 48.4%, intermediate in 42.2%, and low in 9.4%. Pre-transplant status: 75% MRD-negative, 14.1% MRD-positive, and 10.9% with active disease. Overall 2-year OS was 59%, with median follow-up not reached. Progression-Free Survival (PFS) at 2 years was 52%. OS by MRD status: 71% (MRD-negative), 44% (MRD-positive), and 0% (active disease), p=0.0013. OS by ELN risk: 100% (low), 66% (intermediate), and 41% (high), p=0.019. Non-Relapse Mortality (NRM) at 2 years was 29%; relapse incidence was 25%. Acute GVHD grades I–IV occurred in 28% within 100 days, with 5% being grades III–IV. Chronic GVHD at 2 years was 32%, with 20% extensive forms.

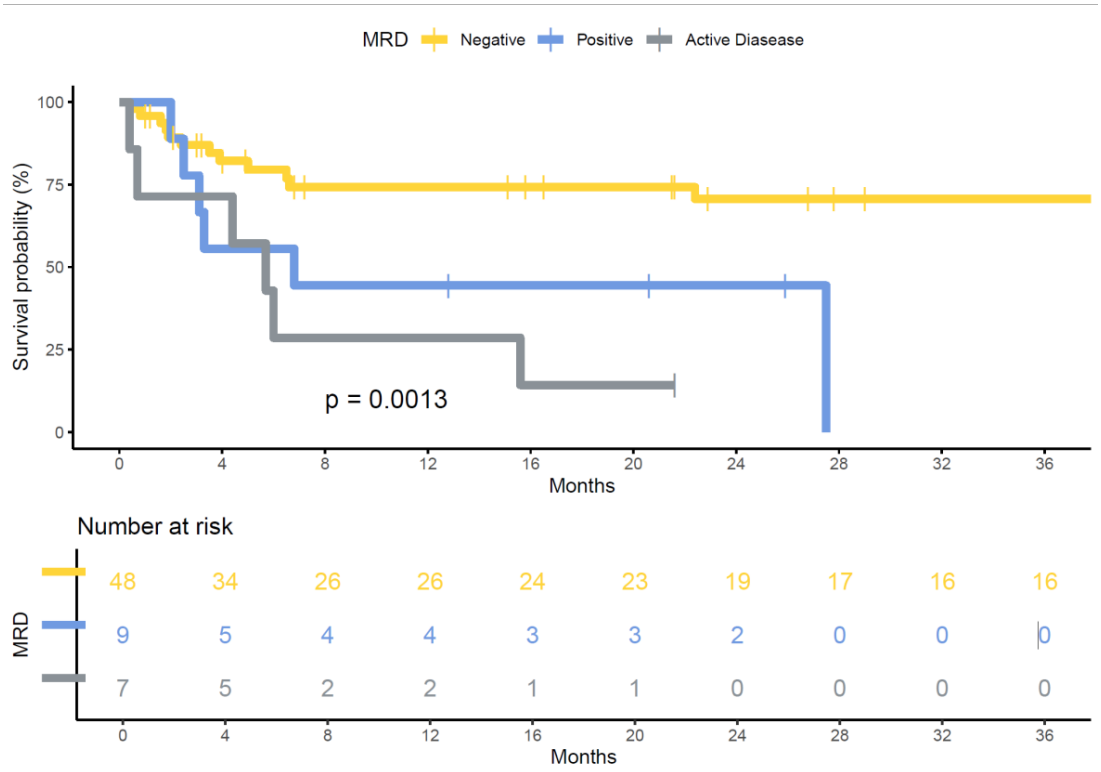
CONCLUSION:

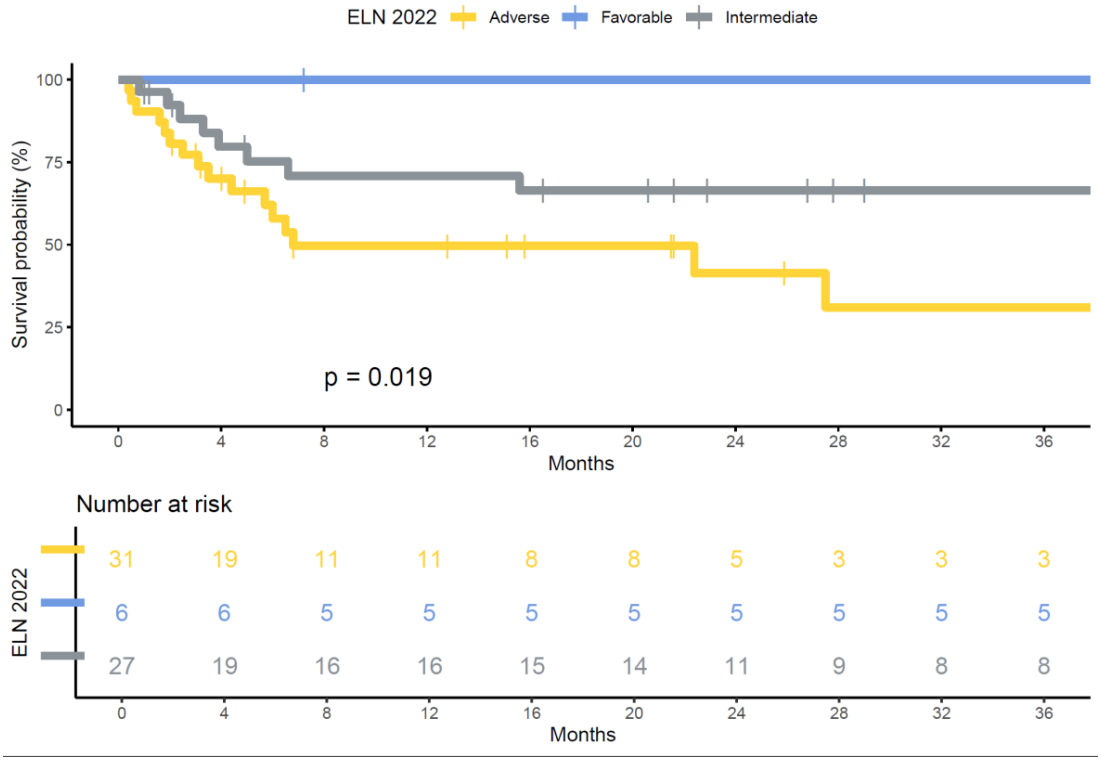
Risk stratification is essential in AML and impacts post-HSCT outcomes. MRD negativity correlates with improved OS, while MRD-positive patients may still benefit from HSCT, with careful consideration of conditioning regimen and toxicity. Active disease at transplant predicts dismal outcomes and should be an exceptional indication.

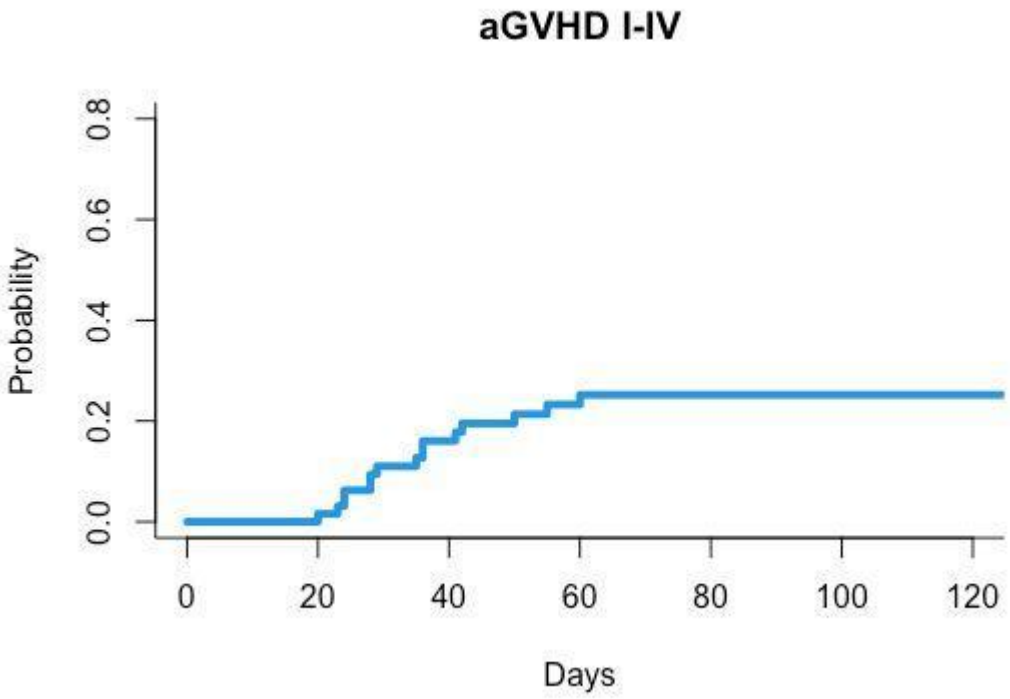
KEYWORDS: Allogenic Bone Marrow Trasplantation; Mensurable Residual Disease; Acute Myeloid Leukemia

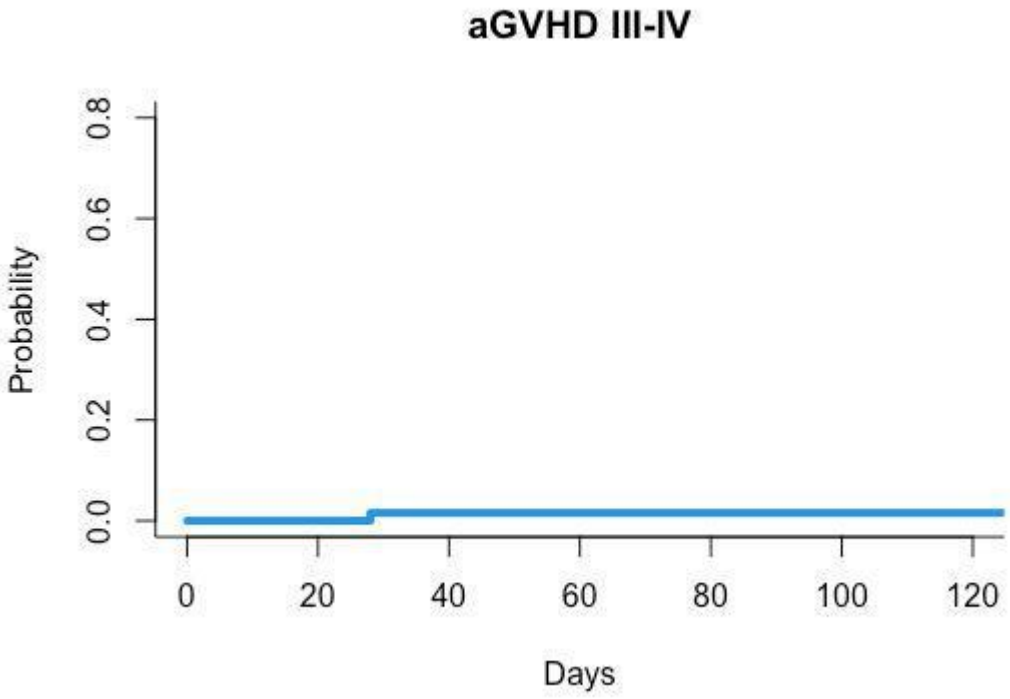

OS
Survival probability (%)
100
75
50
25
0
0 4 8 12 16 20 24 28 32 36
Months
Number at risk
All
64 44 32 32 28 27 21 17 16 16
0 4 8 12 16 20 24 28 32 36
Months

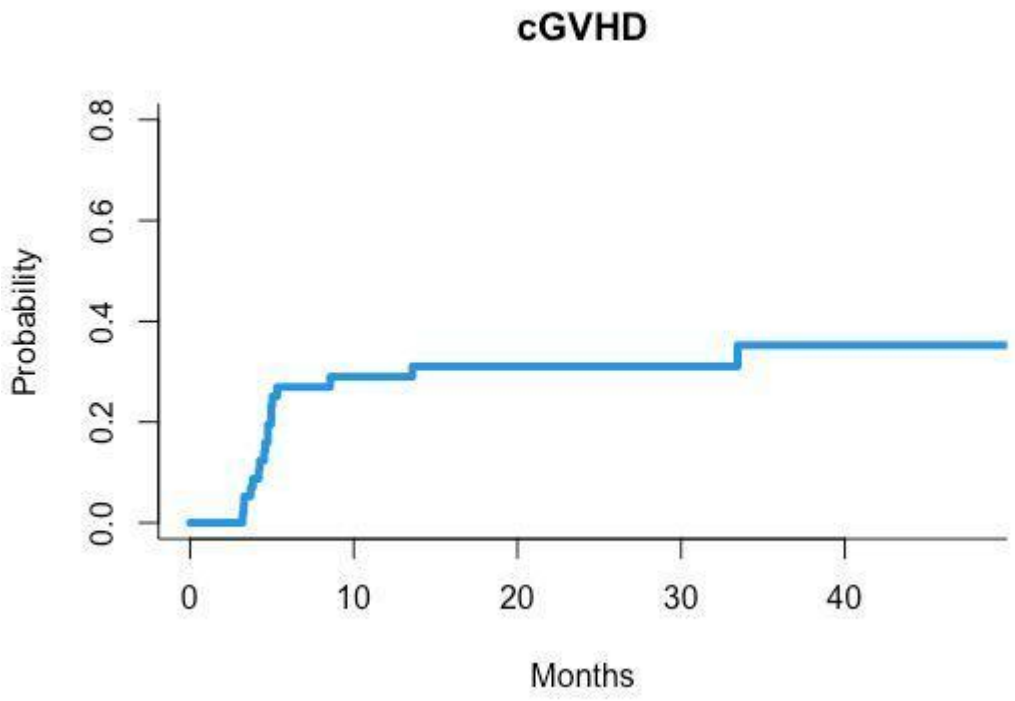

PFS
Survival probability (%)
100
75
50
25
0
0 4 8 12 16 20 24 28 32 36
Months
Number at risk
All
64 42 32 31 28 26 21 17 16 16
0 4 8 12 16 20 24 28 32 36
Months

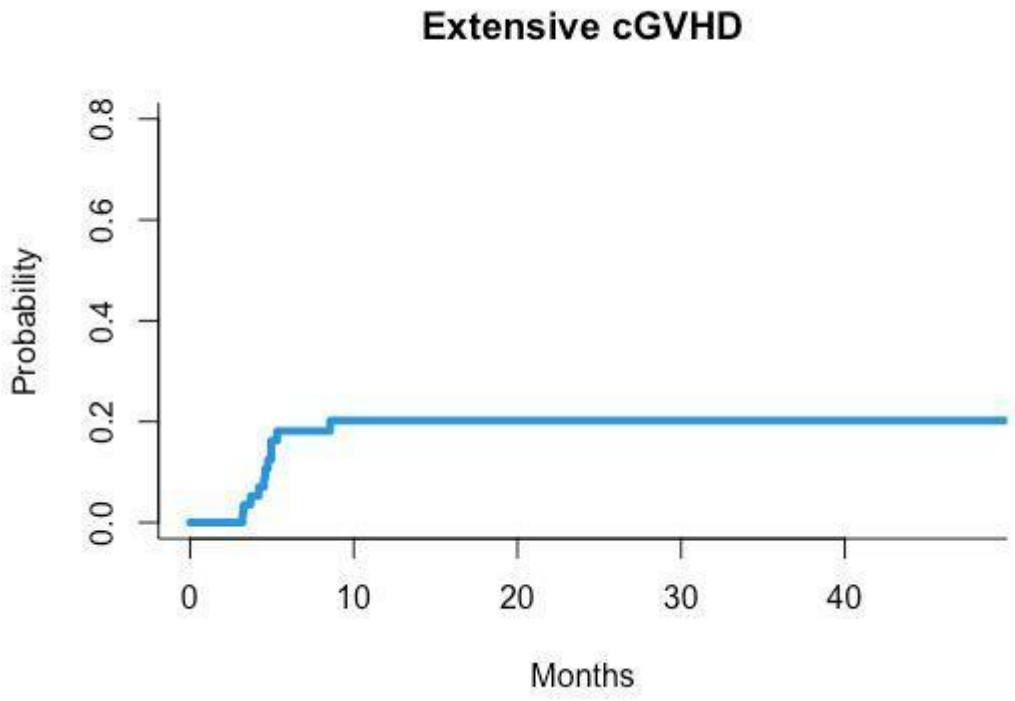

MRD Negative Positive Active Disease
Survival probability (%)
100
75
50
25
0
p = 0.0013
0 4 8 12 16 20 24 28 32 36
Months
Number at risk
MRD
48 34 26 26 24 23 19 17 16 16
9 5 4 4 3 3 2 0 0 0
7 5 2 2 1 1 0 0 0 0
0 4 8 12 16 20 24 28 32 36
Months


ELN 2022 Adverse Favorable Intermediate
Survival probability (%)
100
75
50
25
0
p = 0.019
0 4 8 12 16 20 24 28 32 36
Months
Number at risk
ELN 2022
31 19 11 11 8 8 5 3 3 3
6 6 5 5 5 5 5 5 5 5
27 19 16 16 15 14 11 9 8 8
0 4 8 12 16 20 24 28 32 36
Months


aGVHD I-IV
Probability
0.0
0.2
0.4
0.6
0.8
0
20
40
60
80
100
120
Days


aGVHD III-IV
Probability
0.0
0.2
0.4
0.6
0.8
0
20
40
60
80
100
120
Days


cGVHD
Probability
0.0
0.2
0.4
0.6
0.8
0
10
20
30
40
Months


Extensive cGVHD
Probability
0.0
0.2
0.4
0.6
0.8
0
10
20
30
40
Months

WORSE OUTCOMES WITH MOTHERS AS DONORS FOR PEDIATRIC HAPLOIDENTICAL STEM CELL TRANSPLANTS

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INTRODUCTION:

The number of haploidentical (haplo) hematopoietic cell transplants (HCT) is continuously increasing worldwide, driven by reports of good clinical outcomes. Donor selection remains challenging in face of multiple potential candidates. Criteria considered in donor selection include the presence of anti-donor-specific antibodies (DSA), age, gender, ABO compatibility, KIR haplotype, and immunogenic variables. There is still debate on the impact of gender mismatch in the context of haploidentical female donors to male recipients, that may potentially increase the risk of graft-versus-host disease (GVHD) and HCT-related mortality. This negative impact is attenuated when mother donors are excluded from these analyses.

OBJECTIVE

Evaluate the impact of using mothers as donors for haplo HCT to treat pediatric malignant diseases.

METHODS:

Retrospective review of the medical records of patients undergoing haplo-HCT from January 2021 to December 2024 for the treatment of hematological malignancies.

RESULTS:

64 patients underwent haplo-HCT, with a median age of 8.4 years. All patients had myeloablative conditioning and post-transplant cyclophosphamide (PTCy), calcineurin inhibitor and mycophenolate mofetil for GVHD prophylaxis. Bone marrow was the stem cell source in 75% of the HCT. Most donors were male (62.5%). Among the 24 female donors, 15 were the patients' mothers and, in these cases, the stem cell source was always bone marrow. The incidence of acute GVHD (aGVHD) was 26.5% (11% Magic III/IV) and of chronic GVHD (cGVHD), 40% (28% severe forms requiring systemic treatment). However, HCT with

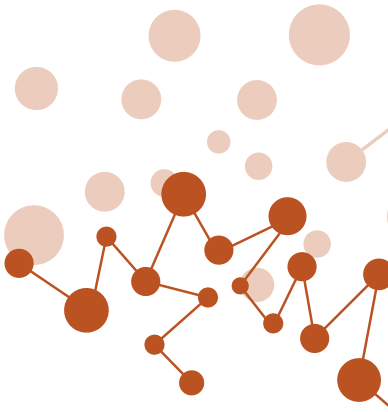
male donors had 25% aGVHD - 10% Magic III/IV, and 35% cGVHD - 20% severe, compared compared with female donors with 29% aGVHD - 2 cases of Magic III/IV, and much higher, 54% cGVHD, 41% severe forms. Among the latter female donors, GVHD has only occurred when the donor was the recipient's mother in all but one patient. Likewise, Magic II/IV GVHD and severe cGVHD were only observed when the mother was the donor despite using marrow grafts. A total of 22 patients have died, 27% of them due to relapse, and the majority due to infections.

CONCLUSION:

In this cohort, the use of the mother as donor was associated with a higher incidence and severity of both acute (p=0.04) and chronic (p=0.0002) GVHD compared to other donor types, including other female donors. Notably, severe GVHD occurred almost exclusively in recipients of maternal grafts, despite the uniform use of PTCy–based prophylaxis and bone marrow as the stem cell source. These findings support previous concerns of using mothers as donors for either male or female recipients. Further multicenter studies are warranted to better delineate the risks and guide donor choice in pediatric haplo-HCT.

KEYWORDS: Haploidentical; Mother; Graft versus disease

CELLULAR THERAPY - HEMOTHERAPY



AUTOLOGOUS CD19 CAR-T CELL THERAPY USING A POINT-OF-CARE MODEL FOR REFRACTORY OR RELAPSED B-CELL NEOPLASMS: A PHASE I CLINICAL TRIAL FROM A BRAZILIAN ACADEMIC CENTER

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INTRODUCTION:

Chimeric Antigen Receptor (CAR) T-cell therapy targeting CD19 shows high efficacy in relapsed/refractory (R/R) B-cell malignancies. However, it faces implementation challenges in low- and middle-income countries due to logistical and manufacturing barriers. This study reports interim results from a Phase I academic point-of-care CD19-directed CAR-T trial manufactured in Brazil.

OBJECTIVE:

To evaluate safety, tolerability, manufacturing feasibility, and early efficacy of autologous CD19 CAR-T therapy in R/R B-cell malignancies.

METHODS:

This unicentric, prospective phase I trial included patients for CAR-T cells therapy, which were manufactured onsite using CliniMACS Prodigy under cGMP. From March 2023 to May 31, 2025, patients were enrolled into 3 cohorts by disease and age (cohort 1, pediatric ALL; 2, adult ALL; 3, adult NHL and CLL) with escalating doses (5e5 to 2e6 CAR+ cells/kg). Following lymphodepletion with FluCy, CAR-T cells were infused per a 3+3 design. Progression-free survival (PFS) and overall survival (OS) were estimated using Kaplan-Meier methodology.

RESULTS:

During the follow-up period, 18 patients were screened, 6 patients were excluded: 4 due to the absence of active disease, 1 due to an active fungal infection, and 1 due to diagnosis of Burkitt lymphoma. With a median follow-up of 11 months, 11 patients were treated (Table 1). Manufacturing success was 100%; median vein-to-vein time 22.3±7.9 days. CRS occurred in 90% (9% Grade 3), ICANS in 45% (27% Grade 3–4), all reversible (Fig. 1). Infections occurred in 8 patients (72%); the most frequent were CMV reactivation and bacterial pneumonia (each 36%). ORR was 81% (95% CI: 48.2–97.7), with CR in 72% (95% CI: 39.0–93.9) (Fig. 2a). Among the 11 infused patients, 7 remain alive and progression-free, including 4 with ALL, 1 with CLL, and 2 NHL. Two patients died: one with NHL experienced early disease progression and died on day +115; another with NHL died from Acinetobacter baumannii sepsis on day +10, precluding response assessment. Among those who were not in CR at the time of data cutoff, 1 patient with NHL had initially achieved CR but relapsed after one year, while a patient with CLL had a partial response (PR), later progressed, and remains alive on day +164. At data cutoff, PFS was 71% (95% CI: 35.0–89.9). OS was 80% (95% CI: 42.4–94.8), and the median OS was not reached (Fig. 2). Regarding

CAR-T cell persistence after infusion, peak expansion assessed by flow cytometry was observed between days 10 and 14 in patients with NHL, between days 7 and 10 in the patient with ALL, and at day 21 in the patient with CLL. CAR-T cells remained detectable for up to 360 days post-infusion.

CONCLUSION:

Academic CD19 CAR-T manufacturing in Brazil is feasible and safe, with promising early efficacy in R/R B-cell malignancies. Toxicities were manageable. These results support further development and broader national implementation in resource-limited settings.

KEYWORDS: CAR T cells. Hematologic Neoplasms. Academic Medical Center.

TABLE 1: Patient Characteristics

Description (N = 11)		Description (N = 11)	
Sex, n(%)		Number of Previous Treatments n (%)	
Female	3 (27.3)	< 2	0 (0)
Male	8 (72.7)	2-3	6 (54.5)
Age, years, median (range)		4-6	4 (36.4)
2 - 17	9	>6	1 (9.1)
≥ 18	42 (9–69)	Viable Infused CAR-T Cells/kg	
Diagnosis, n (%)		0.5 x 10^6	5 (45.4)
ALL	4 (36.4)	1 x 10^6	3 (27.3)
CLL	2 (18.2)	2 x 10^6	3 (27.3)
NHL	5 (45.4)		

Legend: ALL: Acute Lymphoblastic Leukemia; CLL: Chronic Lymphocytic Leukemia; NHL: Non-Hodgkin Lymphoma

FIGURE 1 – Toxicities

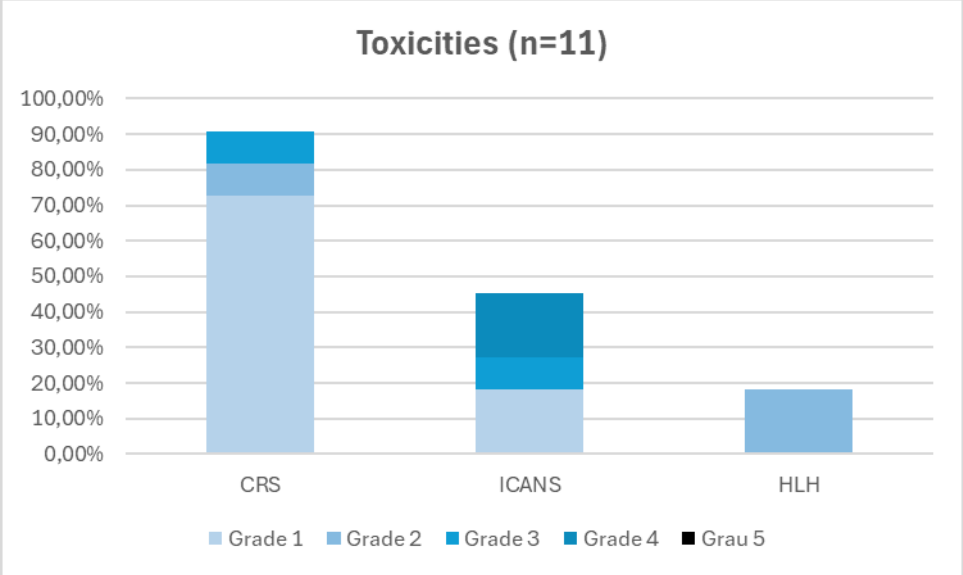


FIGURE 2A – Survival Analysis

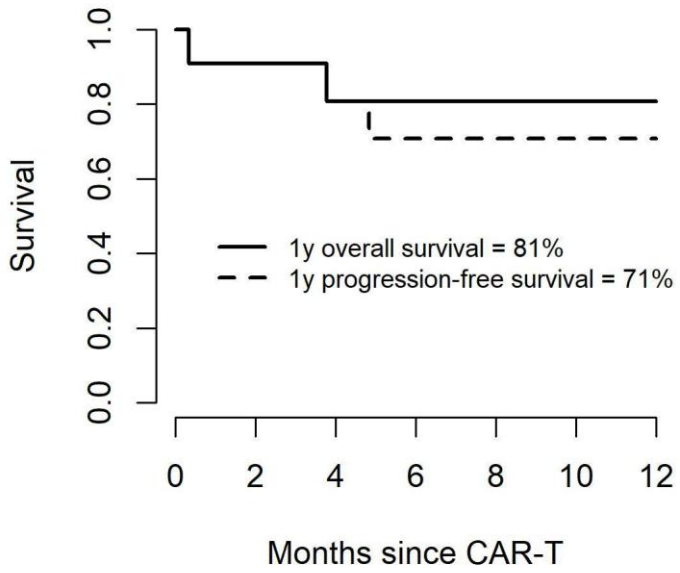
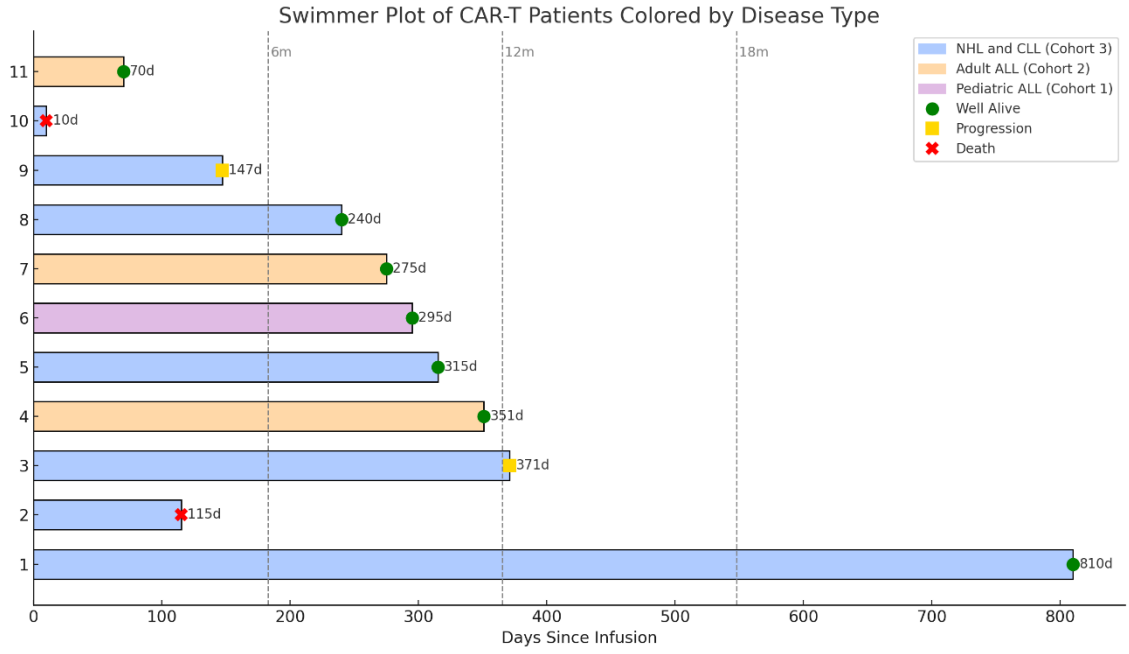


FIGURE 2B – Patients Follow-Up



DISPOSAL OF CPH BAGS: CHALLENGES, STANDARDS AND SUSTAINABILITY

Isabel Aline Fernandes Ferreira[1]; Alexsandra Nunes Pinheiro[1] Marília Silveira Maia[1]; Sâmya Waleska Gomes Nunes[1]; Natércia Maria Moura Bruno[1]; Viviane Aguiar Ferreira Gomes[1]; Vanessa Fernandes Paiva[1]; Weide Barbosa de Mendonça[1]; Luciana Maria de Barros Carlos[1]; Luany Elvira Mesquita Carvalho[1]; Karine Sampaio Nunes Barroso[1]; Fernando Barroso Duarte[1]

1. Centro de Hematologia e Hemoterapia do Estado do Ceará

INTRODUCTION:

The Cell Processing Center (CPC) of a blood center in the Northeast is responsible for the processing, cryopreservation and storage of bags containing Hematopoietic Progenitor Cells (HPC), destined for bone marrow transplantation from 5 transplant centers, four private and one public. All the HPC bags are stored in a -80°C freezer until the time of the transplant. However, in certain situations, it becomes necessary to dispose of these bags, whether due to loss of indication for transplantation, death of the recipient, positive blood culture, clinical decision by the doctor in charge of the transplant center, long storage period or optimization of storage spaces. Disposal of this material is based on the technical instructions that describe the process for disposing of CPH bags, showing compliance with technical and legal requirements. AIM: To report on the experience of disposing of cryopreserved CPH bags as a storage optimization strategy in a Cell Processing Center.

MATERIALS AND METHODS:

Retrospective qualitative and quantitative analysis of the disposal process. The CPH bags were discarded according to criteria pre-established by the CPC: Non-compliance with quality control laboratory tests; Death of the patient; Surplus material not used in transplants; Refusal or discontinuation of treatment for the recipient patient. Discarded bags were recorded on disposal forms, previously signed by the applicant and the technical team responsible for the process, guaranteeing traceability and technical justification. Finally, they were sent to the solid waste sector for incineration, which certifies that the service was carried out.

RESULTS:

A total of 449 CPH bags were discarded between June 2022 and April 2025, from 164 patients, 111 (67.7%) autologous and 53 (32.3%) allogeneic. Of these, 341 (76%) bags were discarded due to cryopreservation time, 99 (22%) bags were discarded due to death, 7 (1.56%) bags were discarded due to a positive blood culture from the same patient and 2 (0.44%) bags were discarded due to a request from the transplant center.

CONCLUSION:

The process was carried out safely, respecting current legislation, the institution's protocols and good practice in the disposal of biological material. The entire process was carried out while maintaining traceability between the sectors involved, and the institutional commitment to patient and staff safety. As of March 2024, patients signed the new Informed Consent Form, which states that products stored for more than three years will be automatically disposed of, except in cases where there is a formal request from the transplant center to extend the deadline

KEYWORDS: HEMATOPOIETIC STEM CELLS; DISPOSAL OF CPH BAGS, SUSTAINABILITY

IN VIVO EVALUATION OF AUTOLOGOUS SERUM EYE DROPS OBTAINED VIA A CLOSED-CIRCUIT COLLECTION DEVICE: PRELIMINARY ANALYSIS OF A PHASE 2 PILOT TRIAL

Giancarlo Fatobene,[1] Luciana Pina,[2] Caio Almeida,[3] Luiz Nani,[3] Joaquim Gasparini,[1] Victoria Sousa,[1] Leonardo Otuyama,[1] Ruth Miyuki Santo,[2] Alfredo Mendrone Júnior,[3] Vanderson Rocha[1]

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INTRODUCTION:

Autologous serum eye drops (ASED) are an established therapy for ocular surface diseases, with reported average symptom improvements of around 50-70%. However, their preparation can be costly and may not be available due to the need for germ-free conditions.

OBJECTIVE:

To evaluate the feasibility and safety of ASED collected via a closed-circuit system device.

METHODS:

This single-arm, Phase 2 pilot trial evaluated a closed-circuit system production of ASEDe. Adult patients with persistent dry eye disease and/or chronic corneal epithelial defects requiring ASED, as evaluated by expert ophthalmologists, were included. 20% ASED were collected, processed, and frozen in single-dose containers (Fig. 1A), which patients thawed and used within 24 hours. Clinical assessments included ophthalmological evaluations (tear film breakup time, meibography, lipid layer analysis, Schirmer test, corneal/conjunctival staining with Rose Bengal and fluorescein), clinical grading (ocular NIH score), symptom questionnaires (OSDI, Lee, global score), treatment adherence, and adverse event monitoring at baseline, 6 weeks, and 12 weeks.

RESULTS:

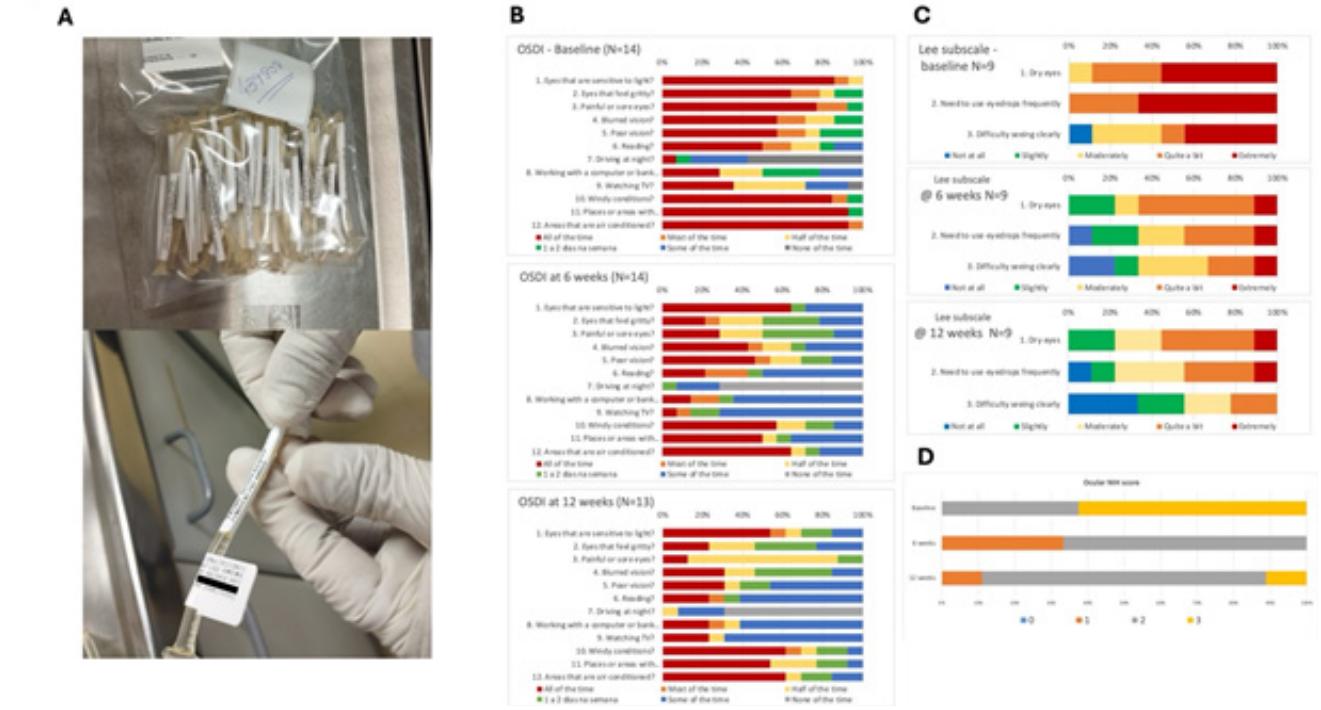
Fourteen patients were enrolled in the pilot phase: 9 with ocular graft-versus-host disease (GVHD), 4 with Sjögren's syndrome, and 1 with cicatricial pemphigoid. As of May 2025, a total of 50 collections have been performed using the closed-circuit system, with three bags lost during centrifugation. No adverse events related to venipuncture or the collection process have been reported. Four patients experienced mild difficulties manipulating the single-dose containers to apply ASED, but adherence remained adequate. No issues were reported with storing containers once opened. All collections met pre-established quality criteria for irregular antibody screening, pre-freezing cultures/cellularity, and pH before and after freezing. Objective ophthalmologic evaluations showed no significant changes from baseline to 6 and 12 weeks post-treatment. Conversely, the OSDI and Lee scores significantly improved over 12 weeks (medians [interquartile range, IQR] 77 [64,92], 53 [33,70], and

45 [25,73], p=.0002 [Fig. 1B]; and 83 [67,100], 50 [42-75] and 58 [42,67], p=.009 [Fig. 1C] at baseline, 6 and 12 weeks, respectively). The ocular NIH score also significantly improved over time (p=.04, Fig. 1D), while the global score showed a trend for improvement (8 [7,10] at baseline, 7 [6,9] at 6 weeks, and 8 [3,8] at 12 weeks, p=.06). Three patients discontinued ASED before completing 12 weeks due to perceived lack of benefit or mild adverse events, which were transient and resolved upon discontinuation.

CONCLUSION:

Preliminary data suggest that closed-circuit system production of ASED appears feasible and safe. Patients experienced symptomatic relief, while objective ophthalmic measures remained stable. This pilot study has been used in the approval for a registration trial under ANVISA, which is set to commence enrollment shortly.

Figure 1:



PERIODIC REVALIDATION AND REQUALIFICATION PROCEDURES PERFORMED AT A CELL PROCESSING CENTER IN SOUTHERN BRAZIL

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INTRODUCTION:

According to current legislation and international quality standards for Cell Therapy Services, cell or tissue banks must perform periodic quality reviews to verify the consistency of the existing process. To achieve this objective, revalidation and requalification actions are carried out. There is no definition of what to revalidate and requalify, since operations and facilities vary considerably in size and complexity. This assessment must be made by each establishment, based on criticality and risk.

OBJECTIVES:

To define which processes and equipment and/or instruments must undergo periodic revalidation and requalification and their frequency.

METHODS:

Based on the definition of the equipment's criticality and the assessment of the direct impact of the process on product quality, the need and frequency of periodic revalidations and requalifications were established. Additionally, all processes performed at this cell processing center are validated upon implementation, and performance qualifications are performed on new inputs and equipment or whenever there is corrective maintenance.

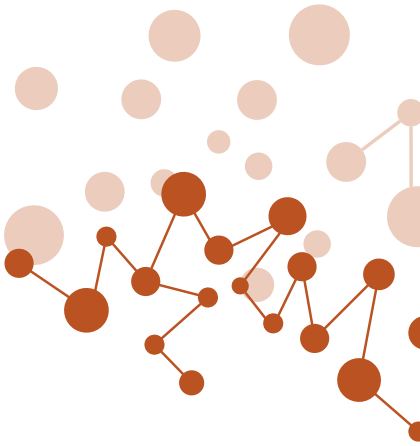
RESULTS:

According to the analysis performed by this cell processing center, the following processes were considered critical and eligible to undergo a periodic revalidation process every two years: plasma reduction of hematopoietic progenitor cells from mobilized peripheral blood (HPC(A)) and bone marrow (HPC(M)), cryopreservation of HPC(A) and HPC(M), red blood cells reduction and plasma reduction after red blood cells removal of HPC(M), and DMSO removal. Revalidation of the bag weights used by the cell processing center must be performed annually. Regarding the annual periodic requalification, the following equipment were defined and selected: sealers, soil centrifuge, dry shippers, micropipettes and freezers -80°C. The hematology counter, the CO2 incubator and the portable temperature recording control devices were included for requalification every two years.

CONCLUSION:

Periodic revalidations and requalifications have ensured, over time, the maintenance of quality and reliability obtained in the implementation of processes and/or equipment considered critical. The defined frequency has proven effective in ensuring the quality of the products supplied by our Cell Processing Center. In the event of any changes to processes and/or equipment, a new reassessment will be promptly performed to ensure continued compliance and safety of the procedures.

ACADEMIC LEAGUES



EVALUATION OF POST-TRANSPLANT CYCLOPHOSPHAMIDE FOR PROPHYLAXIS OF GRAFT-VERSUS-HOST DISEASE: A SYSTEMATIC REVIEW

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INTRODUCTION:

Graft-versus-host disease (GVHD) is a significant complication in allogeneic hematopoietic stem cell transplantation (HLA-matched HCT). Cyclophosphamide prevents GVHD without impairing engraftment by inducing apoptosis in activated alloreactive T cells. Since a standardized GVHD prophylaxis protocol remains undefined, this study aims to evaluate the outcomes of post-transplant cyclophosphamide in its prevention.

OBJECTIVE:

To assess the efficacy of post-transplant cyclophosphamide in preventing graft-versus-host disease in hematopoietic stem cell transplant recipients.

METHODS:

A systematic literature review was conducted according to the PRISMA guidelines for systematic reviews. Article selection was performed through PubMed, Scopus, and Web of Science databases, filtering for studies published in the last five years. The search strategy used the following MeSH terms: "Cyclophosphamide," "Hematopoietic Stem Cell Transplantation," and "Graft vs Host Disease." Studies in Portuguese, English, and Spanish were included. The selected studies underwent risk of bias assessment using the Cochrane Risk of Bias tool (RoB 2).

RESULTS:

A total of 4,907 articles were identified, and after applying inclusion and exclusion criteria, 9 studies were selected. These studies evaluated the efficacy of post-transplant cyclophosphamide (PTCy), either alone or in combination with anti-thymocyte globulin (ATG), for GVHD prophylaxis in allogeneic transplantation. Regarding acute GVHD (aGVHD), PTCy prophylaxis significantly reduced grade II–IV aGVHD rates, ranging from 11.5% to 30%, compared to 39.3% to 50% in control groups ($P<0.05$ in five studies). Grade III–IV aGVHD rates ranged from 6.3% to 7.5% in PTCy groups versus 14.7% to 24.6% in controls ($P<0.05$). For chronic GVHD (cGVHD), a lower incidence was observed with PTCy, with rates between 13% and 24.2%, compared to 26.7% to 39.9% in standard groups, reaching statistical significance in four studies. Overall survival (OS) and disease-free survival (DFS) rates did not differ significantly between the groups. Additionally, a lower incidence of Epstein-Barr virus (EBV) reactivation was noted in PTCy groups (9.8% to 15.1%) versus 60.8% to 82% in conventional prophylaxis groups ($P<0.001$). These findings reinforce the efficacy of PTCy in preventing GVHD without compromising survival or increasing relevant infectious toxicities.

CONCLUSION:

Post-transplant cyclophosphamide proves to be an effective strategy for preventing both acute and chronic GVHD, mitigating its clinical and quality of life impacts without significantly reducing or increasing overall survival. Further studies on optimal PTCy dosing and its combination with other prophylactic strategies may help refine the management of this serious post-transplant complication.

KEYWORDS:

Post-Transplant Cyclophosphamide; Graft-versus-Host Disease; Allogeneic Hematopoietic Stem Cell Transplantation; GVHD Prophylaxis; Systematic Review.

OUTCOMES IN PATIENTS WITH MULTIPLE MYELOMA AND RENAL IMPAIRMENT UNDERGOING AUTOLOGOUS HEMATOPOIETIC CELL TRANSPLANTATION

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INTRODUCTION:

Renal impairment (RI) is a risk factor for reduced overall survival (OS) and increased non-relapse mortality (NRM) in multiple myeloma (MM). While high-dose melphalan followed by autologous hematopoietic cell transplantation (AHCT) is the standard first-line therapy for eligible MM patients, the efficacy and safety of AHCT in patients with RI remain unclear in resource-constrained settings.

OBJECTIVE:

To evaluate the toxicity and efficacy of AHCT in MM patients with RI.

METHODS:

This single-center retrospective cohort study was conducted at a public hospital, including patients with MM and RI (serum creatinine > 2 mg/dL of creatinine clearance < 40 mL/min/1.73m^2 within one month prior to transplant) who underwent AHCT from Jan 2010 to Dec 2023. Grade 3-4 toxicity (NCI-CTCAE 4.0), NRM, progression-free survival (PFS), OS, and the evolution of RI were calculated. Univariate analyses were performed for each outcome. Results: Most patients had advanced MM and two or more comorbidities. Twenty-four patients (46%) received bortezomib, and 32 patients (68%) received immunomodulatory drugs prior to AHCT (Table 1). Grade 3-4 oral mucositis, diarrhea, nausea, and vomiting occurred in 15 (29%), 9 (17%), 7 (13%), and 1 (2%) patients, respectively. Partial remission vs. complete/very good partial remission at AHCT (Odds ratio [OR] 3.94, p=.02) and low albumin (OR 5.55, p=.02) were associated with higher overall grade 3-4 toxicity; a melphalan total dose > 140 mg/m2 showed a trend toward increased toxicity (OR 4.36, p=.053). Febrile neutropenia occurred in 62% of patients, and 26% required intensive care unit (ICU) support (23% received vasoactive drugs and 19% underwent mechanical ventilation). 30-day and 100-day NRM were 9% (95% CI 3-19) and 13% (95% CI 6-24), respectively (Fig. 1). No covariate was significantly linked to NRM. The 24-month PFS and OS were 48% (95% CI 36-66%) and 65% (95% CI: 52-80%), respectively. ISS II or III vs. I (HR 5.05, p=.02; HR 4.53, p=.02) and chronic dialysis (HR 2.22, p=.035) were associated with worse PFS. A Charlson comorbidity index ≥ 3 (HR 2.7, 95% p=.015) was associated with worse OS. Fifteen patients were dialysis-dependent at the time of HCT; all but one remained dialysis-dependent post-transplant, while three died of NRM. Five non-dialysis dependent patients required dialysis during their hospital stay; of these, three remained dialysis-dependent and two were lost to follow-up.

CONCLUSION:

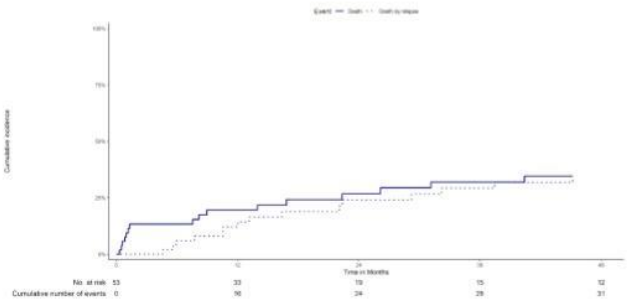
In this resource-constrained setting, AHCT in patients with RI was associated with high NRM and frequent utilization of intensive support, despite the use of lower melphalan doses. These data prompted a change in our institutional protocol to optimize patient selection for HCT and to decrease the melphalan dose to 100 mg/m2 in dialysis-dependent patients.

KEYWORDS: Multiple myeloma, Renal impairment, Autologous hematopoietic cell transplantation

Table 1. Patient, disease and transplant characteristics

Characteristic	N = 53
Gender, N (%)	
Female	34 (64%)
Median age at HCT, years old (IQR)	59 (52, 63)
Pre-HCT response, N (%)	
CR/VGPR	33 (63%)
PR	19 (37%)
Unknown	1
Durie Salmon, N (%)	
IIA/B	9 (18%)
IIIA/B	41 (82%)
Unknown	3
International Staging System, N (%)	
I	7 (13%)
II	12 (23%)
III	33 (63%)
Unknown	1
ECOG at transplant, N (%)	
0-1	37 (76%)
≥ 2	12 (24%)
Unknown	3
Charlson index at transplant, N (%)	
≤ 2	18 (34%)
≥ 3	35 (66%)
Number of treatment lines previously, N (%)	
1	30 (58%)
2	16 (31%)
3	5 (9.6%)
5	1 (1.9%)
Use of immunomodulator prior to transplant, N (%)	
Yes	32 (68%)
Use of proteasome inhibitors prior to transplant, N (%)	24 (46%)
Use of corticosteroids in high doses to transplant, N (%)	49 (94%)
CD34+ infusion, x10^6/kg, median (IQR)	4.01 (3.16, 5.15)
Estimated creatinine clearance*, mL/min/1.73 m^2, median (IQR)	
Before HCT	32 (23, 38)
Any dialysis prior HCT, N (%)	20 (
Chronic dialysis at HCT, N (%)	15 (28%)
Total melphalan dose, N (%)	
100-140 mg/m^2	43 (81%)
>140-200 mg/m^2	10 (19%)
Follow-up, months, median (IQR)	24 (8, 62)

Figure 1. Cumulative incidence of non-relapse mortality



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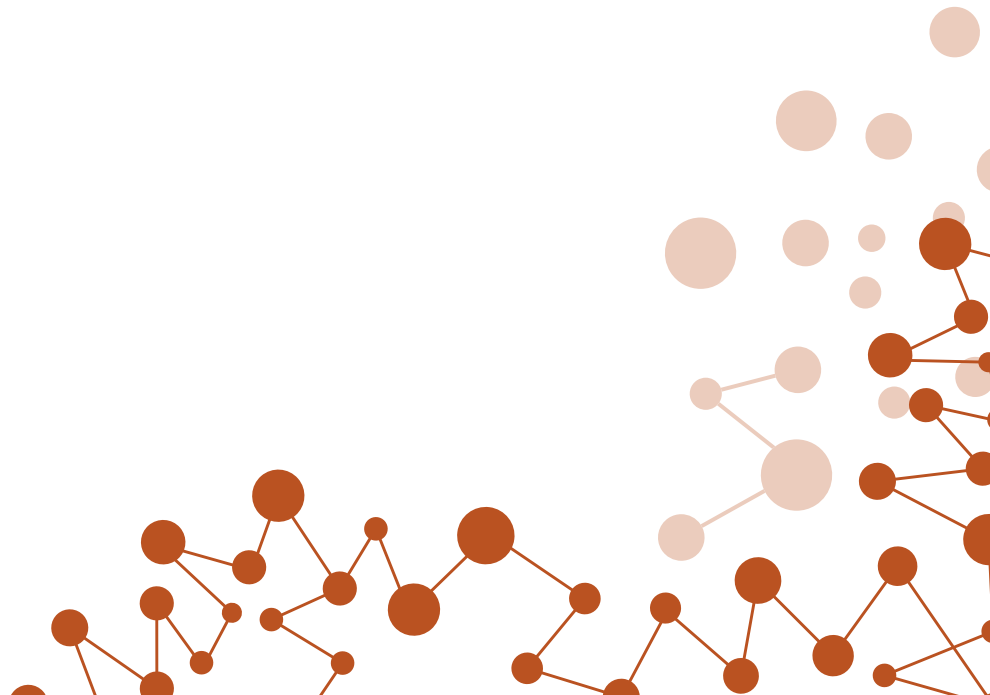

ALLOGENEIC BONE MARROW TRANSPLANTATION FOR SICKLE CELL DISEASE IN A RESOURCE CONSTRAINED SETTING: OUTCOMES AND CHALLENGES

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INTRODUCTION:

In the public healthcare system, allogeneic hematopoietic stem cell transplantation (HSCT) remains the only curative treatment for sickle cell disease (SCD). While initially restricted to pediatric patients, advances in conditioning regimens and graft-versus-host disease (GvHD) prophylaxis have expanded its feasibility to adolescents and young adults.

OBJECTIVES:

To describe the clinical characteristics, transplant-related outcomes, and complications in a cohort of patients with SCD undergoing HLA-identical HSCT.

METHODS:

This retrospective analysis included 20 patients with SCD who underwent HLA-identical HSCT between 2018 and 2024 at a public transplant center in Brazil. Data regarding demographics, transplant indications, graft characteristics, conditioning regimens, complications, and survival outcomes were collected and analyzed.

RESULTS:

The median follow-up was 32.6 months. The median age at transplantation was 15.5 years, with 55% of patients being male. The primary indications for HSCT were recurrent vaso-occlusive crises, acute chest syndrome and neurological complications (Figure 1). All patients received bone marrow grafts; ABO incompatibility was present in 35% of cases. Myeloablative conditioning with fludarabine, busulfan, and rabbit anti-thymocyte globulin (BluFlu + ATG) was used in 19 patients. The GVHD prophylaxis regimen consisted of ciclosporin from D-1 onwards, and methotrexate administered on D+1, D+3, and D+6. Median CD34 cell dose was 2.45 $\times$ 10^6/kg, and median total nucleated cell dose was 2.94 $\times$ 10^8/kg. Median time to neutrophil and platelet engraftment was 20 days. No cases of sinusoidal obstruction syndrome were observed. However, there were two cases of posterior reversible encephalopathy syndrome (PRES). Cytomegalovirus (CMV) reactivation occurred in 11 patients (55%), requiring antiviral treatment for a median of 15 days. Median donor chimerism was 94% (range: 89.6–97%) at day +30 and 91% (range: 84–97%; 95% CI) at day +100. Acute GvHD grades II–IV developed in 9 patients (45%), including 4 with grade II and 5 with grade III. Among patients under 12 years of age (N=9), 3 experienced grade II–IV acute GvHD. Two deaths occurred during follow-up: one due to acute chest syndrome after presenting with secondary graft failure, and the other due to central nervous system infection by human herpesvirus 7 (HHV-7). The overall survival rate at 2 years was 94.7% (95% CI: 85.2–100%, Figure 2).

CONCLUSIONS:

HLA-identical HSCT with myeloablative FluBu + ATG conditioning offers a curative approach for patients with sickle cell disease, demonstrating sustained donor engraftment and acceptable rates of graft failure. However, the incidence of acute GvHD remains significant, particularly in adolescents and young adults. These findings highlight the need for optimized GvHD prophylaxis strategies to improve transplant safety and outcomes in this population.

KEYWORDS: Sickle Cell Disease; HLA-Identical transplant.

Table 1: Patients and HSCT characteristics

Characteristic	N = 20[1]
Sex	
Female	9 (45.0%)
Male	11 (55.0%)
BMI, median (IQR)	18.4 (16.1-20.4)
Age, yr, median (IQR)	15.5 (10.5-20.0)
HbS pre-HSCT, %	44.4 (32.2-55.6)
Sickle cell genotype	
HbSS	16 (80.0%)
HbSC	2 (10.0%)
HbS beta thalassemia	2 (10.0%)
ABO incompatibility	
Absent	13 (65.0%)
Major	2 (10.0%)
Minor	3 (15.0%)
Bidirectional	2 (10.0%)
Donor age, yr, median (IQR)	18.0 (9.5-25.5)
Days to neutrophil engraftment, median (IQR)	20.0 (16.5-24.0)
Days to platelet engraftment, median (IQR)	20.0 (17.0-25.0)

[1] Data presented as median (IQR- interquartile range) or percentages (%)

Figure 1. Indications for HSCT

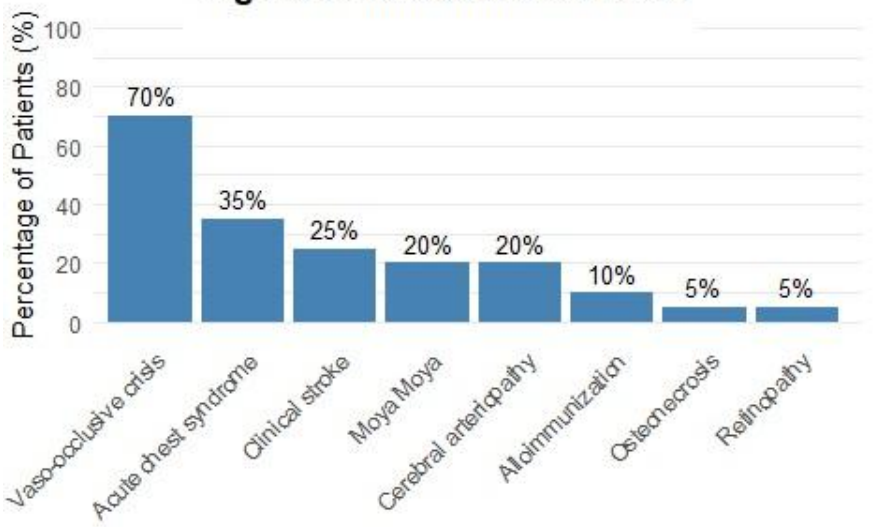
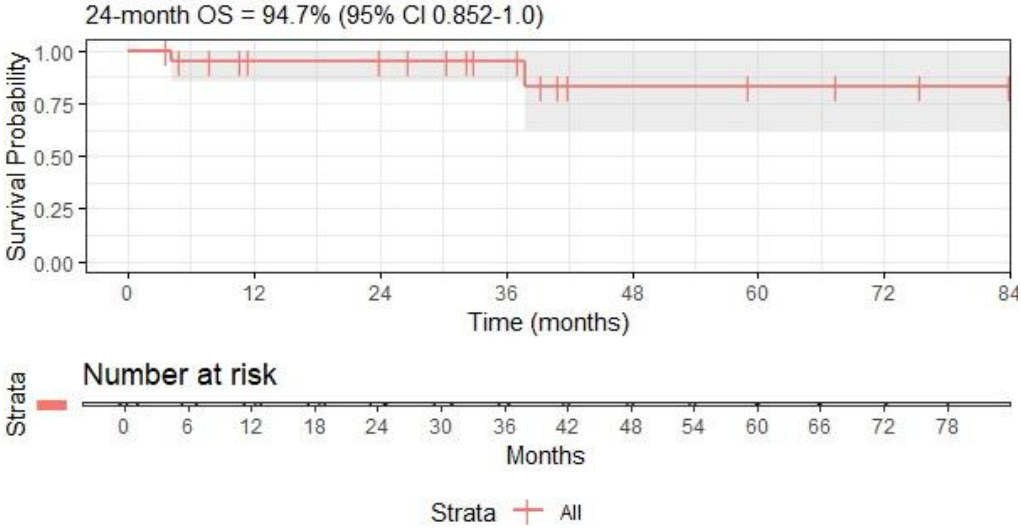


Figure 2. Overall Survival (OS)



ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION IN MYELODYSPLASTIC SYNDROME: IMPACT OF IRON OVERLOAD AND EARLY POST-TRANSPLANT COMPLICATIONS ON SURVIVAL

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1. Hospital de Clínicas de Porto Alegre
2. Universidade Federal do Rio Grande do Sul

INTRODUCTION:

Myelodysplastic syndromes (MDS) are clonal hematopoietic disorders with variable risk of leukemic transformation. Allogeneic hematopoietic stem cell transplantation (HSCT) is the only potentially curative option, but its outcomes are influenced by patient-related and transplant-related factors, especially in low- and middle-income countries. Objective: To describe clinical characteristics and transplant outcomes of MDS patients undergoing HSCT and to identify factors associated with survival.

METHODS:

Retrospective observational study including adult patients with MDS who underwent HSCT between 2004 and 2024 in a Brazilian public tertiary hospital. Demographic, clinical, and transplant-related variables were analyzed. Survival estimates were calculated using the Kaplan–Meier method, and Cox regression was used for multivariate analysis.

RESULTS:

Thirty-three patients were included, with a median age of 46 years and a predominance of females. Most were classified as high-risk by the Revised International Prognostic Scoring System. Bone marrow was the most used graft source (56%), and conditioning regimens were equally distributed between myeloablative and reduced-intensity. The median overall survival was 84 months, with a 5-year survival probability of 35%. Infectious complications were the leading cause of early death, accounting for 60% of cases. Severe mucositis occurred in 40%, with reduced incidence in transplants performed after 2016. Acute and chronic GVHD occurred in 63% and 49%, respectively. Pre-transplant iron overload (ferritin >1000 ng/mL) was significantly associated with inferior survival (median 6 months; HR 2.94;IC 95%: 1,01–8,58; p = 0.048). Donor type, conditioning intensity, and CMV reactivation were not associated with significant differences in survival.

CONCLUSION

HSCT remains a curative strategy for MDS, but early mortality remains high, mainly due to infections. Pre-transplant iron overload is an independent predictor of poor survival and should be addressed during pre-transplant evaluation. These findings highlight the need for improved infection control, supportive care, and iron management strategies to optimize outcomes in MDS transplant recipients.

KEYWORDS: Myelodysplastic syndromes, Allogeneic hematopoietic stem cell transplantation, Iron overload.

ALLOGENEIC HEMATOPOIETIC CELL TRANSPLANTATION: A 10-YEAR EXPERIENCE AT A REFERENCE CENTER IN NORTHEASTERN BRAZIL

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INTRODUCTION:

Allogeneic hematopoietic cell transplantation (HCT) is an immune-based therapy capable of curing malignant hematological diseases and bone marrow failures. The complex processes pose a challenge within the public health system.

OBJECTIVE:

To describe overall survival and relapse-free survival outcomes of HCTs performed at a public tertiary university hospital in northeastern Brazil.

MATERIALS AND METHODS:

A retrospective analysis of medical records of patients who underwent allogeneic HCT between 2014 and 2024. Statistical analysis was performed using R software (version 4.5.0), employing the Kaplan-Meier method for survival analysis.

RESULTS:

A total of 265 transplants were performed during this period, including 177 with related donors, 52 with unrelated donors, and 36 haploidentical transplants. The median age for the procedure was 38 years. The main indication was acute myeloid leukemia (n = 78), followed by acute lymphoblastic leukemia (n = 74), myelodysplastic syndrome (n = 33), and aplastic anemia (n = 32). The overall survival for related donor transplants was 54% and 47% at 5 and 10 years, respectively. Five-year overall survival was 34% for unrelated donors and 39% for haploidentical transplants. Median survival was 8.8 years for related donor, 1 year for unrelated, and 1.8 years for haploidentical transplants. When evaluating survival by indication, 1-year overall survival was 75% for acute myeloid leukemia, 62% for acute lymphoblastic leukemia, 62% for aplastic anemia, and 66% for myelodysplastic syndrome.

DISCUSSION:

Outcomes in allogeneic HCT are influenced by several factors, including donor type, disease status at the time of transplant, conditioning intensity, age, presence of donor-specific antibodies, and cytomegalovirus serostatus. Timely access to transplantation provides a better chance for survival. In the context of the public healthcare system, several challenges persist, such as the availability of adequate hospital infrastructure; access to total body irradiation and prophylactic medications for infection; therapies to eliminate measurable residual disease pre-transplant and prevent post-transplant relapse; as well as effective treatment for steroid-refractory graft-versus-host disease.

CONCLUSION:

Allogeneic transplantation offers the possibility of cure and prolonged survival for many patients with hematological diseases. Expanding access and optimizing outcomes in experienced transplant centers are important goals for public health.

ASSESSMENT OF CELL VIABILITY AS A COMPLEMENTARY CRITERION FOR SAMPLE ACCEPTABILITY IN CELL SUBSET CHIMERISM TESTING: INSIGHTS FROM AN EDUCATIONAL CHALLENGE AND EXPERIMENTAL ANALYSIS

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INTRODUCTION:

The American Society for Histocompatibility and Immunogenetics (ASHI) Proficiency Testing Program recently introduced an educational challenge for subpopulation chimerism. In 2024, we chose to participate in this initiative, performing subpopulation chimerism testing targeting T lymphocytes and the myeloid lineage, despite the samples exceeding the recommended 48-hour stability period.

OBJECTIVE:

This study aimed to assess the impact of processing time on cell subset separation and to investigate whether cell viability could serve as a marker of sample acceptability for subpopulation chimerism analysis.

METHODS:

Cell subset separation was performed on four proficiency testing (PT) samples using immunomagnetic selection with monoclonal antibody-coated beads specific for CD3 (T lymphocytes) and on four PT samples using beads specific for CD33 (myeloid lineage), according to the manufacturer's protocol (StemCell Technologies). The purity of the isolated cell populations was confirmed by flow cytometric analysis. Chimerism analysis was conducted using short tandem repeat (STR) profiling with the GlobalFiler™ PCR Amplification Kit (Thermo Fisher Scientific). Data were analyzed using ChimerMarker® software (SoftGenetics). Cell viability (CD45-positive/7AAD-negative cells) and cell processing were assessed on the day of collection and after seven days using a peripheral blood sample from a patient with myelodysplastic syndrome.

RESULTS:

The laboratory achieved 100% concordance in the official PT samples. However, discrepancies were observed in four myeloid subset chimerism results from the educational challenge samples. The observed chimerism percentages were as follows: sample 1 – 56% (consensus: 49.8%), sample 2 – 4% (consensus: 3.4%), sample 3 – 32% (consensus: 11%), and sample 4 – 90% (consensus: 81.5%). These differences were noted despite cell purity exceeding 90% in all cases.

Considering that cell viability may be affected by the interval between sample collection and processing, we hypothesized that differences in the proportion of viable cells between donor and recipient could impact chimerism results. A marked reduction in cell viability was observed on day 7 compared to the day of collection (97.37% vs. 63.10%), while cell purity remained above 90% at both time points. Regarding chimerism, results were concordant in the myeloid subset (99% donor chimerism), but discrepant in the lymphoid subset (43% on the day of collection vs. 56% on day 7).

CONCLUSION:

Based on the discrepancies observed in the ASHI educational challenge, cell viability may represent a relevant additional parameter for defining sample acceptability. Its inclusion could enhance analytical quality standards in chimerism testing, particularly in laboratories that process samples from external centers. Further studies are needed to validate this hypothesis.

KEYWORDS: cell viability, subpopulation, chimerism

ASSESSMENT OF EASIX SCORE IN THE CLINICAL PRACTICE OF ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION

Allogeneic Hematopoietic Stem Cell Transplantation (HSCT) is an effective treatment for various hematologic diseases. However, post-transplant outcomes are variable and predictive scores are limited. The high non-relapse mortality associated with this treatment is one of the main clinical challenges, and its prediction is based on recipient's pre-existing comorbidities, specific risks of the underlying disease and donor-related factors. Risk assessment based on the quantification of endothelial dysfunction prior to HSCT appears to be a promising approach to help predict post-transplant mortality. The Endothelial Activation and Stress Index (EASIX) is one way to assess endothelial activation and has been correlated with other biomarkers of endothelial dysfunction. In allogeneic HSCT, this index appears to be predictive of toxicity, non-relapse mortality, and overall survival. Objective To assess whether patients who underwent HSCT with pre-EASIX ≥ 3 had higher treatment-related mortality.

METHODS

Retrospective study conducted from January 2008 to December 2023, including 252 patients over 18 years of age who underwent HSCT.

RESULTS

124 (49.2%) allogeneic HSCT recipients had a pre-transplant EASIX score ≥3. Overall survival, general mortality, and non-relapse mortality were all higher in the group with an EASIX score ≥3 (p=0.001).

CONCLUSION

The results of this study have validated standard laboratory biomarker index for estimating post-transplant mortality risk. A pre-conditioning EASIX score ≥3 identifies a population of allogeneic HSCT recipients with more than twice the risk of treatment-related mortality.

KEYWORDS: Allogeneic Hematopoietic Stem Cell Transplantation, Endothelial dysfunction, Post-transplant mortality risk.

ASSOCIATION BETWEEN INFUSED CD34⁺ CELL DOSE AND THE DEVELOPMENT OF GRAFT-VERSUS-HOST DISEASE

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INTRODUCTION:

The impact of infused CD34⁺ cell dose has been investigated as a potential modulator of outcomes following allogeneic hematopoietic stem cell transplantation (alloHSCT). Although there are minimum thresholds required to ensure adequate engraftment, the effect of high CD34⁺ doses on graft-versus-host disease (GVHD) remains controversial. Some studies suggest higher doses increase immune reactivity, while others report no significant correlation.

OBJECTIVE:

Assess association between infused CD34⁺ dose and grade III-IV acute (MAGIC) or moderate/severe chronic (NIH) GVHD within 100 days post-alloHSCT.

METHODS:

This single-center, retrospective, observational and analytical study included patients with malignant diseases who underwent alloHSCT using peripheral blood stem cell grafts. Statistical analysis was performed in GraphPad Prism (v10.04), with p-values < 0.05 considered significant.

STUDY POPULATION:

Eighty-one patients (≥18 years) were included and stratified by infused CD34⁺ dose (≤5×10⁶/kg vs. >5×10⁶/kg). Baseline characteristics were similar between the groups (Table 1). GVHD outcomes were analyzed in the 77 patients alive on day +100 post-transplant.

RESULTS:

Within the 77 evaluated patients, the overall incidence of grade III-IV acute GVHD (aGVHD) was 16.9%. Specifically, aGVHD was observed in 3 of 22 patients (13.6%) who received a CD34⁺cell dose ≤5×10⁶/kg and in 10 of 55 patients (18.2%) who received a dose >5×10⁶/kg. There was no statistically significant association between CD34⁺ cell dose and the incidence of aGVHD ($p = 0.75$). The calculated hazard ratio (HR) was 0.77 (95% CI: 0.26–1.90), and the odds ratio (OR) was 0.71 (95% CI: 0.19–2.53), indicating no clinically relevant protective or deleterious effect associated with higher cell doses. In relation to moderate/severe chronic GVHD (cGVHD), the overall incidence was 10.4%, with 1 case (4.5%) occurring in the ≤5×10⁶/kg group and 7 cases (12.7%) in the >5×10⁶/kg group. Again, no statistically significant difference was detected ($p = 0.43$). The HR was 0.43 (95% CI: 0.07–1.70), and the OR was 0.34 (95% CI: 0.02–2.21), reinforcing the absence of a significant association between CD34⁺cell dose and the risk of cGVHD.

CONCLUSIONS:

This study did not demonstrate a significant association between $CD34^+$ cell dose and grade III-IV acute and moderade/severe chronic GVHD development in the first 100 days after alloHSCT. Notably, doses $>5\times10^6$/kg were not linked to increased GVHD incidence, reinforcing their safety. Moreover, higher doses may help reduce the duration of post-transplant aplasia and promote faster hematologic recovery without increasing the risk of GVHD. Conversely, donor type, ABO incompatibility, age, and sex could be associated with clinical outcomes and may inform future risk stratification strategies.

KEYWORDS: Graft-versus-host disease; Allogeneic hematopoietic stem cell transplantation; $CD34^+$ cells; Risk stratification

TABLE 1: Clinical characteristics of patients with acute and chronic GVHD and their association with CD34+ cell dose

Total of patients N = 81		≤ 5x10⁶/kg (22)	≥ 5x10⁶/kg (59)	p-value
Age (years)[1]	43.2	43.7 ± 14.3	44.9 ± 14.2	0.71
Male (%)	55	9 (40.9%)	36 (61.0%)	0.13
Conditioning (%)				1.0
MAC	35	13 (59.1%)	38 (64.4%)	
RIC	46	9 (40.9%)	21 (35.6%)	
Donor type (%)				0.66
MRD	38	11 (50.0%)	27 (45.8%)	
MUD	15	5 (22.7%)	10 (16.9%)	
Haplo	28	6 (27.3%)	22 (37.3%)	
Grade III-IV acute GVHD[2]	35	9 (40.9%)	26 (44.1%)	0.8
Moderate/severe chronic GVHD[3]	18	4 (18.2%)	14 (23.7%)	0.76

1- Mean ± standard deviation; 2- We considered only patients diagnosed with grade III and IV aGVHD (according to the MAGIC classification); 3- We considered only patients diagnosed with moderate and severe cGVHD (according to the NIH classification); MAC: myeloablative conditioning; RIC: reduced-intensity conditioning; MRD: matched related donor transplant; MUD: matched unrelated donor transplant; Haplo: haploidentical related donor transplant.

BUSULFAN/FLUDARABINE VERSUS BUSULFAN/ CYCLOPHOSPHAMIDE AS MYELOABLATIVE CONDITIONING FOR ALLOGENEIC HEMATOPOIETIC CELL TRANSPLANTATION IN PATIENTS WITH ACUTE MYELOID LEUKEMIA: A CASE-CONTROL STUDY

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INTRODUCTION:

Allogeneic Hematopoietic Stem Cell Transplantation (HSCT) represents the treatment of choice for eligible patients with Acute Myeloid Leukemia (AML) classified as intermediate- or high-risk.

OBJECTIVE:

To conduct a retrospective case-control study comparing the outcomes of two myeloablative conditioning regimens: Busulfan/Fludarabine (BuFlu) and Busulfan/Cyclophosphamide (BuCy). Hypothesis: The study aims to demonstrate the equivalence between the two conditioning regimens with respect to overall survival (OS), disease-free survival (DFS), non-relapse mortality (NRM), and relapse incidence.

Patients and Methods: This was a retrospective, observational, case-control study based on the review of medical records of patients who underwent allogeneic HSCT between 1994 and July 2023 at the Hematopoietic Stem Cell Transplantation Unit of the University of Campinas.

RESULTS:

No significant differences were observed between the BuFlu and BuCy groups regarding the number of red blood cell and platelet transfusions, length of hospital stay, use of parenteral nutrition, incidence of bacterial and fungal infections, cytomegalovirus reactivation, hepatic veno-occlusive disease, or mucositis. Neutrophil engraftment occurred significantly earlier in the BuFlu group (71.83% vs. 83.33%, P = 0.03), whereas no significant difference was observed for platelet engraftment. The incidence of both acute and chronic graft-versus-host disease did not differ significantly between the groups. Similarly, no significant differences were found in OS, DFS, or cumulative incidence of NRM. However, the BuFlu group exhibited a significantly higher relapse rate (50% vs. 15.56%, P = 0.01).

CONCLUSION:

The BuFlu conditioning regimen was associated with faster neutrophil engraftment and a higher cumulative incidence of relapse compared to BuCy. No significant differences were observed in other clinical outcomes, including survival and non-relapse mortality.

KEYWORDS: Conditioning regimen, Acute Myeloid Leukemia, Myeloablative conditioning

CASE STUDY: HLA COMPATIBILITY IN A BONE MARROW TRANSPLANT FOR A PATIENT WITH FANCONI ANEMIA AND A RELATIVE

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INTRODUCTION:

Fanconi anemia is a rare and heterogeneous genetic disorder caused by mutations in genes related to the regulation of DNA repair, with an incidence of 1 in 360 thousand births. The only definitive/curative treatment for this disease is hematopoietic stem cell transplantation.

OBJECTIVES:

Therefore, the aim of this case study was to investigate the HLA compatibility between a 4-year-old patient with Fanconi anemia phenotype and her grandmother.

METHODS:

A family study of possible compatible bone marrow donors was requested, which included the mother, sister, 10 maternal and paternal uncles, and one paternal grandmother. For this purpose, an EDTA tube was taken from each person, and the DNA samples were extracted using the Biopur kit and the PCR-SSO method was performed using One Lambda kits.

RESULTS:

As a result, genotypes A*02 A*66, B*35 B*41, DRB1*11 DRB1*13, DQA1*01 DQA1*05 and DQB1*03 DQB106 were found for the patient. The genotypes A02 A31, B35 B51 were found in the mother and one uncle, A*31 A*66, B*41 B*51 in the sister and three uncles, A*29 A*66, B*41 B*44 in four uncles and A*02 A*29, B*35 B*44 in two aunts. The genotypes A*02 A*66, B*35 B*41, DRB1*11 DRB1*13, DQA1*01 DQA1*05 and DQB1*03 DQB1*06 were found in the paternal grandmother.

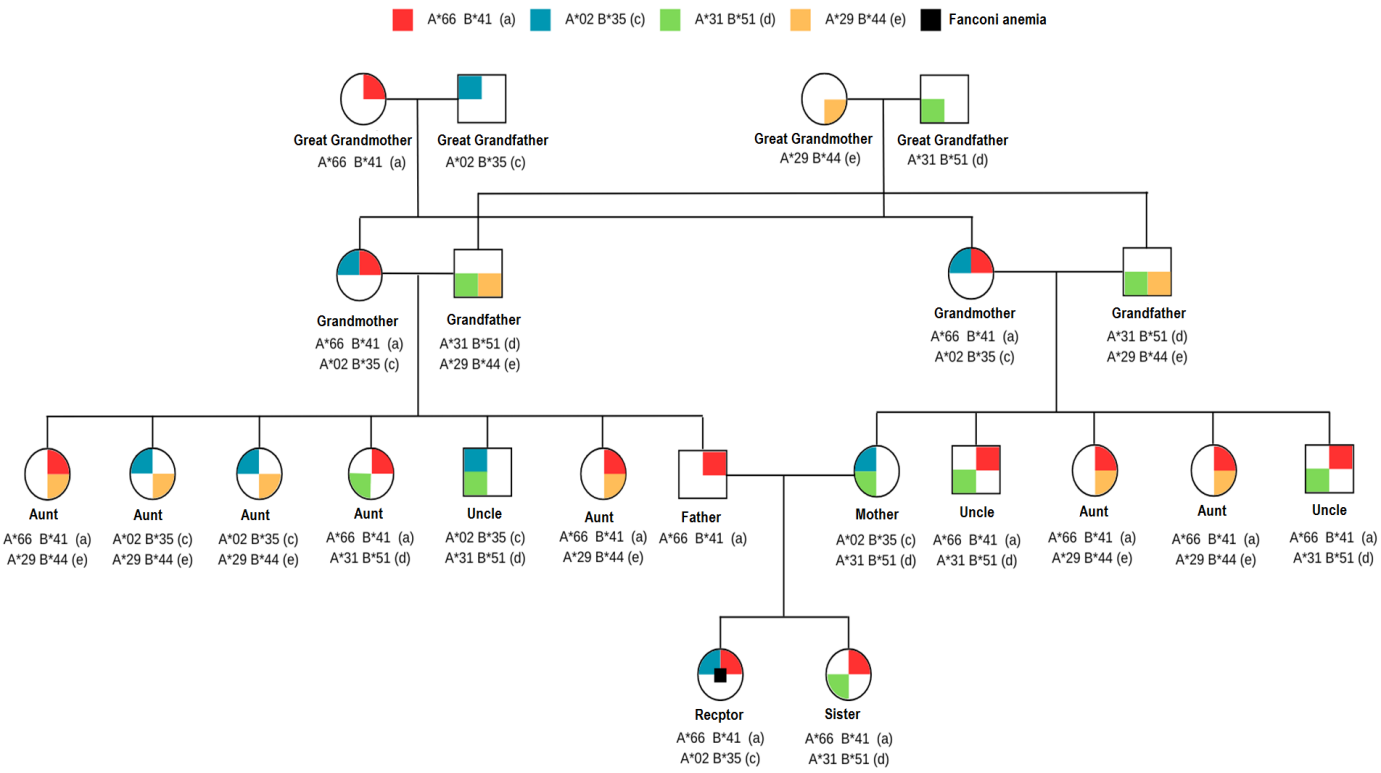
CONCLUSION:

The family investigation revealed that the patient's grandmothers are sisters and her grandfathers are brothers, so the mother and uncles are first cousins. The paternal grandmother is therefore HLA-identical to the patient, and the mother, sister and uncles are HLA-haploidentical to the patient. The probability of finding an HLA-identical donor is 25% for siblings. For family members who are not siblings, the probability is low and occurs mainly in consanguineous marriages. In the patient's case, there was consanguinity on both her mother's and father's side, which increases the probability of finding an HLA-identical donor.

KEYWORDS:

HLA, Fanconi anemia, case study.

FIGURE 1 Family pedigree



CHALLENGES IN THE USE OF MOLECULAR INTERNATIONAL PROGNOSTIC SCORING (IPSS-M) IN THE DECISION-MAKING FOR HEMATOPOIETIC CELL TRANSPLANTATION IN MYELODYSPLASTIC SYNDROMES (MDS) IN LATIN AMERICA.

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INTRODUCTION:

The risk stratification in Myelodysplastic Syndrome (MDS) is crucial for therapeutic decision-making and the Molecular International Prognostic Scoring System (IPSS-M) model was recently developed incorporating molecular data. However, access to molecular testing and availability of molecular data in Latin America (LA) are heterogeneous, mainly due to high costs and limited reimbursement.

OBJECTIVES:

The aim of the study was to evaluate the risk stratification by IPSS-M, clinical implications, and prognosis of patients with MDS in the Latin American Registry.

METHODS:

This retrospective registry study included 450 MDS patients who received an allogeneic hematopoietic cell transplantation (HCT) from the transplant registry of 38 centers in LA between 2016 to 2023. For multiple comparison analyses, the Chi-square test, Fisher's Exact Test, and Kruskal-Wallis test were conducted. The Kaplan-Meier curve estimated survival probability over time. Statistical analyses were performed using SPSSv23.1, with $p<0.05$ as the significanc.

RESULTS:

Of the 450 patients, 58% were male. Median age was 46 years (2-82). The frequency of death was (38%). The IPSS-R risk stratification, most patients were in the intermediate (22.89%) and high-risk (19.56%) groups at the time of diagnosis, while very low (0,44%), low (8,89%), and very high risk (6.67%) groups were less prevalent. However, 41.6% of patients were unclassified according to IPSS-R risk groups.The frequency of stratification according to the IPSS-M during the study period was 6.88%. Between 2019 and 2024, this frequency increased to 12.75%, with an additional increase between 2022 and 2024, reaching 28.4%. In the IPSS-M stratification, patients were classified into the following risk groups: very low (13.33%), low (6.68%), moderate low (13.33%), moderate high (13.33%), high (40%), and very high (13.33%). The IPSS-M re-stratified 66% of patients (Figure 1A), with 40% up-staged and 26% down-staged. A study conducted in South America reported similar findings, with 44% of patients up-staged and 17% down-staged (Lincango et al., 2023)(Figure 1B). The 5-years overall survival (OS) rate was 54.5%. No significant difference in OS was observed between patients with and without molecular examination ($p=0.081$)(Figure 2).The IPSS-M was associated with the transfusion of more than 20 units of red blood cells ($p=0.02$) and more than 15 units of platelets ($p=0.025$), previous treatment with hypomethylating ($p<0.001$), reduced-intensity conditioning ($p=0.01$), absence of infection ($p=0.035$), MUD and HID donor types ($p=0.030$).

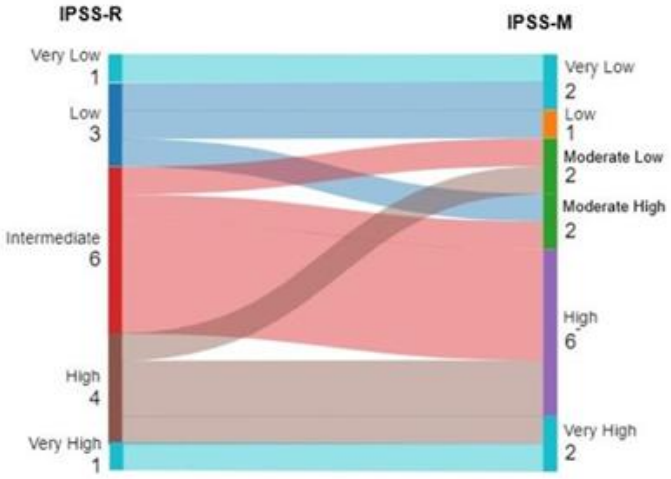
CONCLUSION:

The IPSS-M showed an association with important clinical outcomes. In our sample, patients who underwent molecular analysis had greater access to hypomethylating agents and other therapies, which may represent a source of bias. Although molecular testing showed a trend towards better OS, further research will be needed to demonstrate its prognostic accuracy in HCT outcomes in LA.

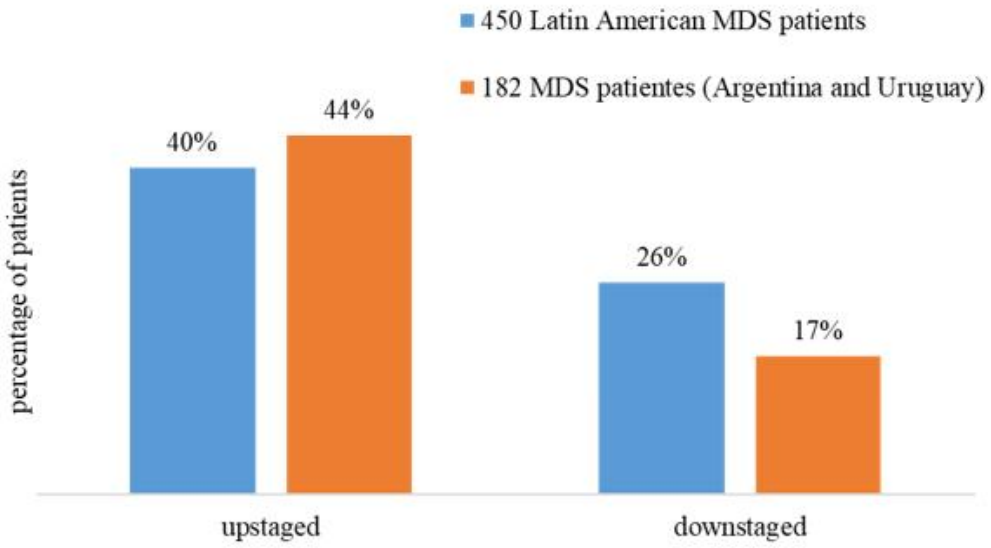
KEYWORDS: allogeneic hematopoietic cell transplantation; Molecular International Prognostic Scoring, myelodysplastic syndrome.

Figure 1: Restratification of IPSS-R to IPSS-M risk groups

A)

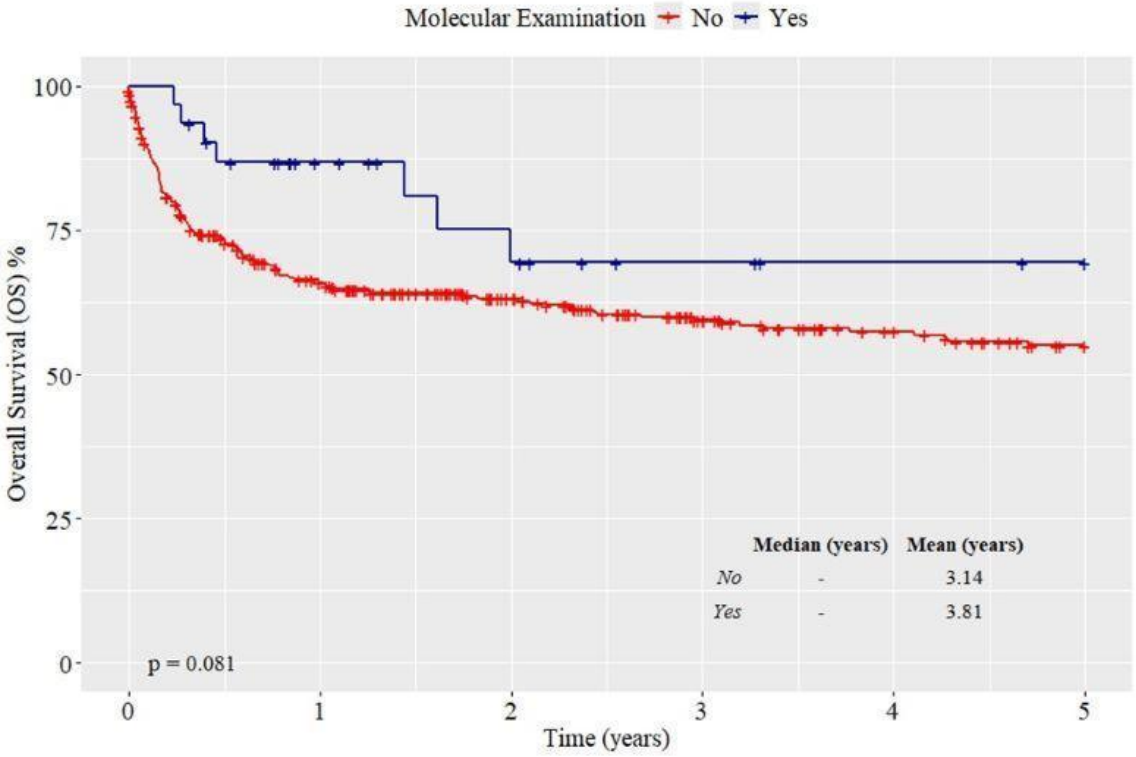


B)



Fonte: Adapted from Lincango et al., 2023.

Figure 2: Overall Survival according to IPSS-M stratification



CLINICAL AND TRANSPLANT-RELATED DETERMINANTS OF ACUTE AND CHRONIC GRAFT-VERSUS-HOST DISEASE: INSIGHTS FROM A SINGLE-CENTER COHORT

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BACKGROUND:

Allogeneic hematopoietic stem cell transplantation (HSCT) is a curative therapy for hematological disorders but is often complicated by graft-versus-host disease (GVHD), a major cause of morbidity and mortality. GVHD manifests in acute and chronic forms, each characterized by distinct risk factors and pathophysiological mechanisms. Understanding these factors is essential for improving patient outcomes and guiding prophylactic strategies.

OBJECTIVE:

This study aimed to evaluate clinical and transplant-related characteristics associated with the development of acute and chronic GVHD in a single-center cohort.

METHODS:

We retrospectively analyzed 71 patients who underwent allogeneic HSCT between 2022 and 2024 (see Table 1). Among these, 7 patients were pediatric and the remaining were adults. All grafts were obtained via peripheral blood stem cell apheresis. The cohort included 68 related donors whose cells were cryopreserved and 3 unrelated donors whose cells were infused fresh. Patients were grouped into no GVHD (n=34), acute GVHD (n=28), and chronic GVHD (n=9). Variables analyzed included patient and donor age, graft composition ($CD34^+$ and $CD3^+$ cell dose), donor-recipient characteristics, CMV risk, ABO compatibility, diagnosis, transplant type, engraftment time, and mortality. Continuous variables were compared using Kruskal–Wallis with Dunn's post hoc test. Categorical variables were analyzed by Chi-square or Fisher's exact test. A p-value < 0.05 was considered significant.

RESULTS:

Patient age differed significantly across groups (p=0.0266), with younger patients more prone to acute GVHD and older patients to chronic GVHD. $CD3^+$ cell dose was significantly higher in the acute GVHD group (p=0.0460), while patients with chronic GVHD had the lowest T-cell doses. Donors of patients who developed chronic GVHD tended to be older. No significant associations were observed with sex, ABO compatibility, CMV risk, or diagnosis. Haploidentical transplants were frequent across groups. Graft failure occurred in 3 patients in the no-GVHD group and in 1 patient in the acute GVHD group; all resulted in death before day 30. Mortality was observed between days 31–100 in patients with acute GVHD and after day 100 in those with chronic GVHD.

CONCLUSION:

This study highlights the significant impact of patient and donor age, as well as the infused $CD3^+$ cell dose, on the risk and subtype of GVHD following allogeneic HSCT. Younger recipients were more prone to acute GVHD, while older patients tended to develop chronic GVHD. Similarly, older donor age correlated with chronic GVHD incidence. The $CD3^+$ T-cell dose was notably higher in patients who developed acute GVHD, underscoring its role in mediating early alloimmune responses. These results support personalized GVHD prophylaxis based on key clinical and transplant factors.

KEYWORDS:

Hematopoietic Stem Cell Transplantation (HSCT), Graft-versus-Host Disease (GVHD), Clinical Risk Factors

TABLE 1. Clinical and Demographic Characteristics, Transplantation Parameters, and Graft-versus-Host Disease Outcomes of 71 Patients Undergoing Allogeneic Hematopoietic Stem Cell Transplantation Between 2022 and 2024

Variable	No GVHD n=34	Acute GVHD n=28	Chronic GVHD n=9	p value
Patient age, years median (IQR)	55.0 (64.3-42.0)	43.5 (54.3-32.3)	64.0 (73.5-41.5)	*0.0266
Body mass, Kg median (IQR)	71.7 (78.4-63.8)	69.2 (83.6-57.3)	78.0 (85.0-60.5)	
Sex, n (%) Male Female	18 (25.0%) 16 (23.0%)	18 (25.0%) 10 (14.0%)	7 (10.0%) 2 (3.0%)	
Donor age, years median (IQR)	42.0 (54.5-29.0)	35.5 (56.8-26.3)	61.0 (65.5-44.5)	
$CD34x10^6$/kg, median (IQR)	7.6 (9,5-6.2)	7.9 (9.4-6.1)	8.5 (8.8-5.2)	
CD3 x 10^7/kg, median (IQR)	18.9 (26.2-7.2)	23.6 (30.3-11.1)	9.5 (12.8-6.5)	*0.0460
Donor sex, n (%) Male Female	21 (30.0%) 13 (18.0%)	18 (25.0%) 10 (14.0%)	8 (11.0%) 1 (1.0%)	
CMV risk, n (%) Low Moderate High	3 (4.0%) 28 (39.0%) 3 (4.0%)	2 (3.0%) 24 (34.0%) 2 (3.0%)	1 (1.0%) 8 (11.0%) 0 (0.0%)	
ABO compatibility, n (%) Compatible Minor Major Bidirectional	28 (39.0%) 1 (1.0%) 5 (7.0%) 0 (0.0%)	19 (27.0%) 3 (4.0%) 5 (7.0%) 1 (1.0%)	8 (11.0%) 0 (0.0%) 1 (1.0%) 0 (0.0%)	
Diagnosis, n (%) Leukemia, Lymphoma Others	25 (35.0%) 1 (1.0%) 8 (11.0%)	21 (30.0%) 4 (6.0%) 3 (4.0%)	6 (8.0%) 0 (0.0%) 3 (4.0%)	
Transplant type, n(%) Full match (10/10) Haploidentical Mismatch (9/10)	14 (20.0%) 20 (28.0%) 0 (0.0 %)	14 (20.0%) 14 (20.0%) 0 (0.0%)	5 (7.0%) 3 (4.0%) 1 (1.0%)	
Neutrophil Engraftment, days median (IQR)	15 (17-13)	14 (17-14)	13 (20-12)	
Death, n (%) D0-D30 D31-D100 D101-D365	3 (4.0%) 4 (6.0%) 3 (4.0%)	1 (1.0%) 8 (11.0%) 8 (11.0%)	0 (0.0%) 2 (3.0%) 3 (4.0%)	

CLINICAL PROFILE OF FANCONI ANEMIA AND OUTCOMES WITH DIFFERENT CONDITIONING REGIMENS TO HEMATOPOIETIC STEM CELL TRANSPLANTATION IN NORTHEAST REFERENCE CENTERS

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Fanconi anemia (FA) is a rare hereditary disorder marked by chromosomal instability from DNA repair defects. FA patients face heightened risks of hematologic malignancies and other cancers. Severe aplastic anemia leads to high morbidity and mortality due to infections and hemorrhages. Progression to myelodysplastic syndromes (MDS) or acute myeloid leukemia is common, with rare cases of lymphoid leukemias and solid tumors, including head and neck, anogenital, and oral cancers. FA shows marked clinical heterogeneity: about 60% of patients have congenital malformations affecting multiple systems (skeletal, integumentary, urogenital, cardiopulmonary, gastrointestinal, and CNS), while 20% show no distinct phenotype but present with pancytopenia and bone marrow hypoplasia/aplasia. In cases with subtle anomalies, diagnosis often occurs after bone marrow failure, typically between ages five and ten. Post-treatment complications include hemosiderosis from transfusions, virilization from prolonged androgen or corticosteroid use, and issues linked to hematopoietic stem cell transplantation (HSCT). Androgens may offer temporary benefit, but the only curative treatment is allogeneic HSCT, which shows better outcomes in patients with few transfusions and a matched sibling donor—though relapse occurs in ~40% of cases. This study aimed to retrospectively and cross-sectionally analyze medical records of nine FA patients treated at four institutions in northeastern Brazil, all having undergone HSCT at least one year prior. The cohort included five males and four females, aged 10–25. Frequent symptoms were short stature and petechiae (100%), café-au-lait spots (44%), and skeletal anomalies (33%). All had pancytopenia; diagnosis was confirmed via Mitomycin C assay due to limited access to the DEB test. All underwent allogeneic HSCT—one with a related donor, eight with unrelated. The main conditioning regimen was cyclophosphamide + antithymocyte globulin (44%), followed by fludarabine + cyclophosphamide + antilymphocytic globulin (33%), and fludarabine + cyclophosphamide + busulfan (23%). One year post-HSCT, significant hematological improvement was noted, especially in those on the first regimen: hemoglobin rose by 33%, leukocytes by 67%, and platelets from 42,000/mm^3 to 129,000/mm^3—surpassing literature-reported gains of 15–30%. Recent studies highlight the efficacy of regimens like FLU + CFA + GAL in improving hematological outcomes in FA patients.

KEYWORD: Fanconi Anemia, Allogeneic HSCT, hematopoietic stem cell transplantation

COMPARISON OF HLA GENETIC PROFILES BETWEEN RECEPTOR AND VOLUNTEER DONORS REGISTERED IN THE BRAZILIAN BONE MARROW VOLUNTEER DONOR REGISTRY IN MINAS GERAIS

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INTRODUCTION:

Bone marrow transplantation is essential for the treatment of certain diseases, and a compatible donor is required to perform it. There is a 25% chance of finding a compatible donor in the family. However, if it is not possible to find a compatible donor in the family, databases of volunteer bone marrow donors, such as REDOME, must be searched.

OBJECTIVES:

The aim of this study was to compare the genetic variation of the HLA gene in bone marrow receptor between 2018 and 2021 with donors from REDOME in Minas Gerais between August 2023 and March 2025.

METHODS:

In receptor, HLA typing was performed using the PCR-SSO method with One Lambda kits, and in volunteer donors, sequencing of the A, B, DRB1 and DQB1 loci was performed using NGS on the IonTorrent and MGI platforms. The results were analyzed using Fusion 4.2 or TSV 3.0 software. During the analyzed period, 1.102 HLA typing of receptor and 8.250 HLA typing of donors were performed by REDOME.

RESULTS:

The most common genotypes found at loci A in receptor and donors were A*02 with A*02 (4.9 % and 6 %), A*02 with A*68 (4 % and 3.8 %), A*01 with A*02 (4 % and 4.4 %) and A*02 with A*03 (4.3 % and 4.6 %). For locus B, the most common genotypes in receptor and donors were B35 with B44 (2.9% and 2.7%), B15 with B*44 (2.9% and 2.1%) and B*08 with B*44 (2% and 1.1%, p-value < 0.05). At the DRB1 locus, the most frequent genotypes in receptor and donors were DRB1*04 with DRB1*13 (4% and 3.5%), DRB1*04 with DRB1*15 (3.8% and 2.5%, p-value < 0.05), DRB1*03 with DRB1*13 (4.3% and 3.2%), DRB1*11 with DRB1*13 (3.2% and 2.9%) and DRB1*07 with DRB1*11 (3.4% and 2.7%). In addition, the genotypes DQB1*03 with DQB1*06 (14.4% and 13.6%, respectively), DQB1*02 with DQB1*03 (11.9% and 12.8%, respectively) and DQB1*02 with DQB1*06 (10.1% and 10.1%, respectively) were found at the DQB1 locus in receptor and donors. We can therefore conclude that significant differences were found between receptor and donors for the genotypes B*8 with B*44 and DRB1*04 with DRB1*15.

CONCLUSION:

These results highlight the importance of monitoring and understanding the genetic variation between receptor and donors in order to identify regional trends and thus contribute to the efficiency and success of bone marrow transplant programs in Brazil and worldwide.

KEYWORDS: HLA, REDOME, allele frequency.

TABLE 01 - **Common genotypes**

Common genotypes	Recptor		Donors		P-value
	n	%	n	%	
A locus					
A*02 / A*02	54	4,9	522	6	0,089
A*02 / A*68	43	4	310	3,8	0,899
A*01 / A*02	47	4	361	4,4	0,920
A*02 / A*03	47	4,3	379	4,6	0,681
B locus					
B*35 / B*44	32	2,9	221	2,7	0,758
B*15 / B*44	32	2,9	170	2,1	0,101
B*08 / B*44	22	2	93	1,1	0,024*
DRB1 locus					
DRB1*04 / DRB1*13	44	4	290	3,5	0,503
DRB1*04 / DRB1*15	42	3,8	205	2,5	0,017*
DRB1*03 / DRB1*13	47	4,3	262	3,2	0,085
DRB1*11 / DRB1*13	35	3,2	237	2,9	0,663
DRB1*07 / DRB1*11	37	3,4	219	2,7	0,234
DQB1 locus					
DQB1*03 / DQB1*06	159	14,4	1123	13,6	0,571
DQB1*02 / DQB1*03	131	11,9	1059	12,8	0,445
DQB1*02 / DQB1*06	112	10,1	836	10,1	1,000

DESENSITIZATION FOR DONOR-SPECIFIC ANTI-HLA ANTIBODIES ENABLING HAPLOIDENTICAL HSCT IN HIGH-RISK B-ALL: A CASE REPORT

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INTRODUCTION:

B-cell Acute Lymphoblastic Leukemia (B-ALL) is an aggressive hematologic malignancy requiring intensive therapy, especially in patients with high-risk cytogenetic and molecular features. Allogeneic hematopoietic stem cell transplantation (HSCT) remains the only curative option in many such cases. However, the presence of donor-specific anti-HLA antibodies (DSAs) poses a significant challenge, increasing the risk of graft failure and poor graft engraftment.

CASE SUMMARY:

A 62-year-old female was diagnosed in June 2024 with Philadelphia chromosome-negative B-cell ALL with leukemic infiltration of the central nervous system. Cytogenetics showed a complex karyotype: 46XXt(5;13)(q13;q34), t(11;19)(q23;p13-1), and next-generation sequencing revealed an MLLT1 mutation (uncertain significance). She underwent treatment with the HyperCVAD protocol (induction and consolidation), but persistent measurable residual disease (MRD) was detected in bone marrow immunophenotyping. Blinatumomab was initiated in September 2024, achieving complete remission, though with significant neurotoxicity. Given the high-risk disease profile and lack of an HLA-matched donor, haploidentical HSCT was indicated as the only curative approach. Pre-transplant immunological evaluation revealed high-titer donor-specific anti-HLA antibodies (anti-HLA-Cw05 >1500 MFI), necessitating desensitization. The planned desensitization protocol included plasmapheresis on days -14, -12, and -10; Rituximab 375 mg/m^2 (700 mg) on day -8; and donor buffy coat infusion on day -1. Conditioning consisted of Fludarabine (30 mg/m^2, 5 days), intravenous (IV) busulfan (3.2 mg/kg, 2 days) and total body irradiation (400 cGy on day -1). Prior to stem cell infusion, the anti-HLA-Cw05 titer was <300 MFI. GVHD prophylaxis included post-transplant Cyclophosphamide, Cyclosporine, and Mycophenolate Mofetil. She presented neutrophil engraftment on day +20 and platelet engraftment on day +28, with a well-functioning graft to current follow-up. The DSA titer persisted undetected until hospital discharge.

DISCUSSION:

Donor-specific anti-HLA antibodies are a recognized risk factor for primary graft failure and delayed engraftment in HSCT. Desensitization protocols aim to lower DSA titers before infusion of donor stem cells to enhance engraftment success. Strategies typically include plasmapheresis to reduce circulating antibodies, Rituximab to deplete B cells, and buffy coat infusion as an immunologic sink. Monitoring MFI values before and after treatment helps assess efficacy. Therefore, individualized desensitization remains essential, particularly when no matched donor is available.

CONCLUSION:

This case highlights the complex management of high-risk B-ALL with DSA-positive status. Haploidentical HSCT with pre-transplant desensitization offers a viable curative strategy. Timely desensitization protocols are crucial to minimize graft rejection risk and optimize transplant success in sensitized and high-risk patients.

HEMATOPOIETIC PROGENITOR CELL PRODUCTS FROM PERIPHERAL BLOOD AND BONE MARROW: DIFFERENCES IN PARAMETERS

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INTRODUCTION:

There are three sources of hematopoietic progenitor cells (HPC) for transplantation: mobilized peripheral blood (HPC-PB), bone marrow (HPC-BM), and umbilical cord blood (HPC-CB). Each HPC source has characteristic parameters that influence the final outcome of transplantation (HSCT).

OBJECTIVE:

To compare critical parameters of HPC-PB and HPC-BM products for allogeneic use, processed at the Cell Therapy Laboratory of the Ribeirão Preto Blood Center. Subjects: 181 HPC products obtained from 181 donors.

METHOD:

This is a cross-sectional and retrospective study. A total of 181 allogeneic HPC products were analyzed, including 61 HPC-PB and 120 HPC-BM products. The products were compared in terms of the following parameters: total volume, red blood cell (RBC) volume, hematocrit, leukocyte concentration, platelet concentration, total nucleated cell and CD34-cell dose. We also evaluated the microorganism contamination of each product type (BM: 266; PB: 1,733). Data were analyzed using Mann-Whitney and Fisher's exact tests.

RESULTS:

The median (range) values for total volume, RBC volume, and hematocrit were significantly higher in HPC-BM than in HPC-PB products: total volume (mL): 1,042 (249.8 – 1904) vs. 225.8 (98.83 – 469.9); RBC volume (mL): 322.3 (69.37 – 693.1) vs. 5.27 (0.25 – 25.53); hematocrit (%): 36.6 (21 – 40) vs. 2.6 (1 – 12.8) (all with $p < 0.0001$). Conversely, HPC-PB products had significantly higher values for leukocyte concentration, total nucleated cell (TNC), CD34-cell count, and platelet concentration: leukocytes (10^3/µL): 297 (109 – 594) vs. 16.95 (2.8 – 5.2); TNC (total): 689.7 (218.5 – 1,452) vs. 171.3 (45.48 – 420); CD34-cell dose: 522.8 (154.9 – 1,456) vs. 136.7 (37.34 – 384.8); and platelet concentration (10^3/µL): 259 (61 – 707) vs. 76.5 (28 – 258) (all with $p< 0.0001$). Contamination rate in HPC-BM is 16 times higher than that observed in HPC-PB (11.28 vs 0.7%, respectively; $p< 0.0001$). Also, Gram+ bacteria (Staphylococcus epidermidis) predominates in HPC-BM, and Gram- bacteria (Escherichia coli and Salmonella sp) predominates in HPC-PB.

CONCLUSION:

HPC-PB products are characterized by high yield of progenitor cells and TNC, lower risk of microbiological contamination, lower risk of hemolytic reactions in cases of ABO mismatch and faster hematopoietic recovery, as the literature has shown. Nevertheless, allogeneic use of HPC-PB requires prior pharmacological mobilization, which may be associated with adverse effects from G-CSF use in donors. The results suggest that the HPC-PB offers clinical advantages over HPC-BM products.

KEYWORDS: allogeneic HPC transplantation; bone marrow; mobilized peripheral blood.

TABLE 01

HSC-BM X HSC-PB: Products characteristics

median (range)
*Mann-Whitney test

	HSC-BM (n= 120)	HSC-PB (n= 61)	*p
Total volume (mL)	1.042 (249,8 – 1.904)	225,8 (98,83 – 469,9)	< 0,0001
Red blood cell volume (mL)	322,3 (69,37 – 693,1)	5,27 (0,26 – 25,53)	< 0,0001
Hematocrit (%)	31,6 (21 – 40)	2,6 (0,1 – 12,8)	< 0,0001
Leukocytes (x 10^3/µL)	16,95 (2,8 – 5,2)	297 (109 – 594)	< 0,0001
TCN x 10^8 (total)	171,3 (45,48 – 420)	689,7 (218,5 – 1.452)	< 0,0001
CD34 x 10^6 (total)	136,7 (37,34 – 384,8)	522,8 (154,9 – 1.456)	< 0,0001
Platelets (x 10^3/µL)	76,5 (28 – 258)	259 (61 – 707)	< 0,0001

HLA ALLELE FREQUENCY ANALYSIS IN VOLUNTEER DONORS REGISTERED IN THE BRAZILIAN BONE MARROW VOLUNTEER DONOR REGISTRY IN MINAS GERAIS, BRAZIL

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INTRODUCTION:

In Brazil, 5.784.307 voluntary donors are registered in REDOME - Brazilian Registry of Voluntary Bone Marrow Donors - of which 636.172 are in Minas Gerais. According to REDOME, 25.585 donors were registered in Minas Gerais in 2023 and 2024. Objectives: Therefore, the aim of this study was to analyze the frequency of HLA-A, -B, -C, DRB1, DQB1 and DPB1 alleles in REDOME donors in Minas Gerais between August 2023 and March 2025.

METHODS:

Thus, REDOME donor volunteers completed the REDOMEweb registration and took an EDTA tube when participating. In the histocompatibility laboratory, the DNA was extracted and the sequencing of the A, B, C, DRB1, DQB1 and DPB1 loci was performed by NGS using the IonTorrent and MGI platforms. The results were analyzed with the software TSV 3.0 and published on REDOMEweb.

RESULTS:

Between August 2023 and March 2025, 8.250 HLA typings of REDOME donors were performed in Minas Gerais. 66% of the registered donors were female and 34% male, with a predominant age range between 18 and 35 years. This data is consistent with the information available from REDOME on donor gender and age. Regarding ethnicity, 42.7%, 42.7%, and 13.6% self-declared as belonging to the brown, white, and black races, respectively. The following genotypes were frequently found at loci A, B, C, DRB1, DQB1 and DPB1: A*02 with A*02, B*35 with B*44, C*04 with C*07, DRB1*07 with DRB1*13, DQB1*03 with DQB1*06 and DPB1*04 with DPB1*04. The results of this study for loci A, B, C, DRB1, DQB1 and DPB1 are consistent with the results found in the literature: A*02, A*01 and A*03; B*35, B*44 and B*15; C*04 and C*07; DRB1*07, DRB1*13, DRB1*04, DRB1*03 and DRB1*06; DPB1*04, DPB1*02 and DPB1*01, respectively.

CONCLUSION:

Based on the analysis of the genetic variation of the HLA gene of donors from the REDOME of Minas Gerais, it can be concluded that there is a predominance of female donors, reflecting the demographic trends reported by REDOME. In addition, ethnic diversity among donors was significant, with a sizable proportion of self-identified white and multiracial individuals. The genotypes found in greater frequency at loci A, B, C, DRB1, DQB1 and DPB1 confirm the results found in the literature. These results highlight the importance of monitoring and understanding genetic variation to understand regional trends in donor registries and thus contribute to the efficiency and success of bone marrow transplant programs in Brazil and worldwide.

KEYWORDS: HLA, REDOME, allele frequency.

INDIVIDUALIZED SALVAGE THERAPY AND SEQUENTIAL CONDITIONING FOLLOWED BY ALLO-HCT IN RELAPSED / REFRACTORY AML: A CASE SERIES FROM A BRAZILIAN PRIVATE CENTER

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INTRODUCTION:

Allogeneic hematopoietic stem cell transplantation (HSCT) remains the only potentially curative treatment for patients with high-risk myeloid malignancies, despite high relapse rates. Sequential conditioning (SC) after salvage therapy with AML-like induction and reduced-intensity regimens, such as the FLAMSA protocol, has shown promising results. Venetoclax, a BCL-2 inhibitor, has synergistic effects with chemotherapy and minimal added non-hematologic toxicity. This study aimed to describe the characteristics and post-transplant outcomes of patients with high-risk AML undergoing SC and HSCT in a Brazilian private center.

PATIENTS AND METHODS:

We retrospectively analyzed three adult patients with high-risk AML, as defined by ELN 2022 criteria, who underwent SC followed by HSCT between August 2023 and January 2025. All received venetoclax-based salvage therapy and individualized sequential conditioning.

RESULTS:

Patient A, a 45-year-old female, had a complex karyotype and TP53 mutation. After failing 3+7 plus venetoclax, she received decitabine plus venetoclax, followed by matched sibling HSCT using TMI 6 Gy and BuFlu2. GVHD prophylaxis included a calcineurin inhibitor and methotrexate; maintenance included azacitidine, venetoclax, and DLI.

Patient B, a 41-year-old male with NUP98 mutation, monocytic features, and CNS involvement, had primary refractory disease. He received mitoxantrone, cytarabine, venetoclax, CNS-directed radiotherapy (18 Gy), and MADIT, followed by haploidentical HSCT (TMI 6 Gy + BuFlu2 + PTCy), and azacitidine maintenance.

Patient C, a 52-year-old female, had post-HSCT relapsed AML with monosomy 7 and MECOM rearrangement with pre-existing fungal infection. She received mitoxantrone, etoposide, cytarabine, venetoclax, followed by haplo-HSCT (TMI 6 Gy + FluMel + PTCy), with azacitidine and DLI maintenance.

All patients achieved neutrophil engraftment (median: 13 days, range: 12–24) and complete hematologic remission by day +30. No tumor lysis syndrome occurred. Complications included grade III mucositis and febrile neutropenia (grade 2) in all cases. Patient C developed post-transplant BK virus and CMV reactivation. Acute GVHD (grade I–II) was observed in patients A and B, and chronic GVHD (grade II) in patient C. At a median follow-up of 7.2 months, all patients were alive, MRD-negative, and in sustained remission.

DISCUSSION:

This case series demonstrates the feasibility and safety of individualized sequential conditioning including venetoclax, followed by aHSCT in patients with adverse-risk or refractory AML. Despite poor-risk molecular features, all achieved MRD-negative remissions without early relapse or transplant-related mortality.

CONCLUSION:

Sequential therapy incorporating venetoclax followed by HSCT appears feasible and potentially curative in selected patients with relapsed or refractory high-risk AML, including those with primary induction failure or post-transplant relapse.

LONG-TERM (20-YEAR) SURVIVAL FOLLOW-UP OF PEDIATRIC PATIENTS WITH ACUTE LYMPHOBLASTIC LEUKEMIA UNDERGOING TRANSPLANTATION AT A SINGLE CENTER IN RIO DE JANEIRO, BRAZIL

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INTRODUCTION:

Acute lymphoblastic leukemia (ALL) is the most common childhood malignancy, characterized by the uncontrolled proliferation of immature lymphocytes that replace bone marrow and affect hematopoietic and lymphoid organs. Despite advances in chemotherapy and risk stratification, a significant number of patients relapse and require more intensive therapies, such as allogeneic hematopoietic stem cell transplantation (allo-HSCT), which is considered a curative option, especially for relapsed or high-risk patients.

OBJECTIVES:

To evaluate the prognosis of pediatric patients with ALL who underwent allo-HSCT at the National Cancer Institute (INCA) in Rio de Janeiro, Brazil, between 2002 and 2022, focusing on overall survival (OS), event-free survival (EFS), relapse rate, and the occurrence of graft-versus-host disease (GVHD).

METHODS:

This is a retrospective case series study including 190 children with ALL who underwent allo-HSCT at INCA from January 2002 to December 2022. The median age at diagnosis was 7.76 years, and the median time from diagnosis to transplantation was 2.6 years. A total of 91 HLA-matched transplants, 84 semi-matched, and 15 haploidentical transplants were performed. At the time of transplantation, 78 patients were in first complete remission (CR1), 97 in second (CR2), and 15 in third (CR3). The median follow-up time was 120 months. Outcomes included 5-year OS, EFS, relapse rate, and the incidence of acute and chronic GVHD.

RESULTS:

Of the 190 patients, 134 were male. After a median follow-up of 10 years, 91 patients were alive, and 99 had died or had care withdrawn in the terminal stage. Among patients transplanted in CR1, the 5-year OS and EFS rates were 75% and 67%, respectively. The overall 5-year relapse rate was 41.57%. Acute GVHD occurred in 52 cases and chronic GVHD in 43 cases.

CONCLUSION:

Allo-HSCT represents an important therapeutic strategy for pediatric patients with ALL, particularly those in CR1, who showed better survival rates. Relapse remains a major challenge, and GVHD continues to be a significant complication. Long-term follow-up and early interventions are essential to improving outcomes.

KEYWORDS:

Acute lymphoblastic leukemia, Allogeneic hematopoietic stem cell transplantation, Prognosis, Graft-versus-host diseasse, relapsed

MANAGING HIGH-RISK SECONDARY MDS AND POST-TRANSPLANT RELAPSE IN AN ELDERLY PATIENT: A CASE REPORT

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INTRODUCTION:

Myelodysplastic Syndromes (MDS) are myeloid neoplasms characterized by bone marrow failure and risk of progression to acute myeloid leukemia. Modern classification (WHO 2022, ICC 2022) and prognostic scoring systems (IPSS-R, IPSS-M) integrate data for risk stratification and therapeutic decision-making. Allogeneic hematopoietic stem cell transplantation (allo-HCT) is the only curative therapy for high-risk MDS, with reduced-intensity conditioning (RIC) regimens expanding eligibility to older patients. Post-HCT relapse remains a major challenge, often requiring donor lymphocyte infusions (DLI) and second-line therapies.

OBJECTIVE:

To report a case of high-risk secondary Myelodysplastic Syndrome (sMDS) in an elderly patient, highlighting the complex sequence of therapeutic strategies employed, the management of post-HCT relapse, and available salvage options. Methods: Retrospective review of the patient's medical records supported by a scoping review of the literature conducted on PubMed, Scopus.

CASE REPORT:

A 72-year-old female with a history of multiple malignancies previously treated with prolonged cytotoxic chemotherapy regimens was diagnosed with MDS with isolated del(5q) in 2019. A watchful waiting approach was adopted until 2021, when lenalidomide was started. In 2022 the disease progressed to a complex karyotype, with 10-15% bone marrow (BM) blasts on immunohistochemistry (IHC). Her prognostic score was classified as high-risk, with an elevated HCT-Comorbidity Index (HCT-CI). Given the progression, her treatment was switched to venetoclax (VEN)+azacitidine (AZA). In 2023 the patient underwent HLA-identical allo-HCT from her 62-year-old brother. Neutrophil engraftment occurred on day +15, followed by AZA as maintenance therapy.

DISCUSSION:

Years of cytotoxic treatments for multiple malignancies culminated in therapy-related MDS (t-MDS), which carries a poorer prognosis compared to de novo MDS. After refractoriness to lenalidomide, regimen was switched to VEN+AZA, a common salvage strategy. This was followed by allo-HCT with maintenance AZA, associated with a longer time to relapse but has shown no overall survival benefit in trials[5]. Following progressive loss of chimerism post-transplant, despite maintenance, three preemptive DLIs were administered without response, leading to relapse. Second-line therapy was initiated 10 months post-HCT with a therapeutic DLI and VEN+decitabine, which has yielded a sustained response to date. The next potential step for this patient would be a second allo-HCT[7], given her probable eligibility due to a good performance status. Relapse of MDS after HCT occurs in up to 40% of cases; therefore, its occurrence in this patient, despite an optimized and aggressive treatment course, is not unexpected[8]. A potential alternative strategy is prophylactic DLI—administered before any sign of impending relapse—as emerging studies suggest it may have greater efficacy than preemptive DLI[9].

MODERN AND MORE EFFECTIVE GRAFT-VERSUS-HOST PROPHYLAXIS DOES NOT LEAD TO HIGHER RELAPSE RATES: A META-ANALYSIS OF RANDOMIZED CONTROLLED CLINICAL TRIALS

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INTRODUCTION

Graft-versus-host disease (GVHD) continues to be a leading cause of non-relapse mortality (NRM) and long-term morbidity after allogeneic hematopoietic stem cell transplantation (allo-HSCT). However, increased immunosuppression may be linked to higher relapse rates. Over the past decade, the integration of additional immunomodulatory agents has been explored in various transplant platforms.

REVIEW QUESTION

Does more efficient GVHD pharmacologic prophylaxis with immunosuppressive agents lead to a higher relapse rate compared with conventional GVHD prophylaxis in patients who underwent hematopoietic cell transplantation from matched sibling donors or unrelated donors?

METHODS

We conducted a meta-analysis of randomized controlled trials published over the last 12 years. We searched the PubMed and Cochrane databases. Our search strategy was: ((graft versus host disease[tiab]) OR gvhd[tiab]) AND (random*[tiab] OR trial[tiab] OR (phase[tiab] (3[tiab] OR III[tiab] OR three[tiab]))) AND 2013/01/01:2024/12/01[dp] NOT review[PT]. The inclusion criteria included positive results for any GVHD outcome in an HLA-matched setting in studies that compared the addition of another agent. The exclusion criterion was comparisons between two markedly different prophylactic strategies.

RESULTS

After merging the searches from the two databases, a total of 2,646 abstracts were screened, and 27 articles were selected for full-text review. Of those, 20 studies were included in the qualitative analysis, and 15 provided extractable information about relapse rates. The pooled hazard ratio for the relapse rate was 1.13 (95% CI 0.95-1.33; 11 studies; Figure 1A). The pooled risk difference for relapse was 2.01% (95% CI -1.15 to +5.17; 15 studies; Figure 1B).

DISCUSSION

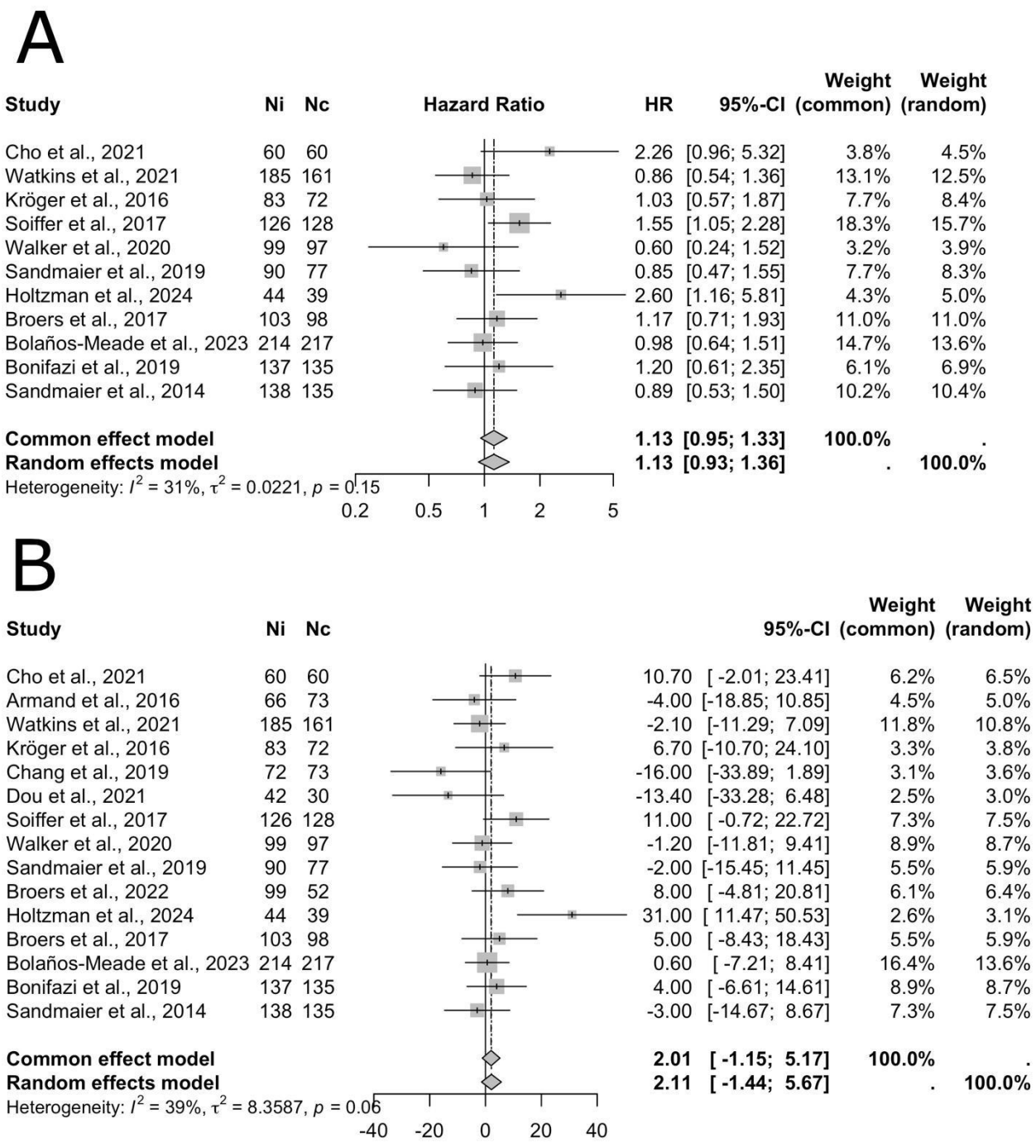
Our results of the pooled analysis of randomized controlled clinical trials with any positive results on GVHD prophylaxis indicate that the relapse rate is not increased, despite improved GVHD control.

CONCLUSIONS

Modern and more effective GVHD prophylaxis should be incorporated into practice for patients undergoing HLA-matched transplants, including those with high-risk disease. This strategy does not increase the risk of relapse.

KEYWORDS: GVHD prophylaxis; relapse; HLA-matched donor

FIGURE 1. Forest plots



Legend: (A) Hazard ratio of relapse, values < 1 favors interventional arm; (B) percentage difference of relapse, values < 0 favors interventinal arm.

MONITORING OF EPSTEIN-BARR VIRUS REACTIVATION IN PATIENTS UNDERGOING ALLOGENEIC HSCT AT THE HOSPITAL DAS CLÍNICAS, HC, UNICAMP, BRAZIL

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ABSTRACT:

The interest in establishing monitoring and treatment strategies for Epstein-Barr virus (EBV) reactivation infection after hematopoietic stem cell transplantation (HSCT) has been growing daily, aiming to propose better early treatment strategies, prevent complications from the infection, and enhance the efficiency of existing care. Given this context, the objective of the present study was to monitor EBV reactivation in patients undergoing allogeneic/haploidentical HSCT at the Hospital de Clínicas (HC), Unicamp, up to day +120 post-transplant, to enable early infection detection, as routine monitoring for these patients is not currently performed in the service. To achieve this, a prospective study was conducted to investigate EBV viral load in five patients who underwent allogeneic/haploidentical HSCT at the Hospital de Clínicas/HC/Unicamp. The allogeneic/haploidentical transplants included identical related donors, identical unrelated donors, and non-identical donors. After obtaining consent, plasma samples were used for EBV detection from day 0 post-HSCT, once a week until day +120. Among the patients included in the study, four out of the five tested showed EBV detection via molecular biology techniques. However, of these four detected cases, only one presented a significant viral load (869 IU/ml), with compatible clinical symptoms, which was confirmed in two subsequent PCR tests. The findings of this study reinforce the importance and great necessity of monitoring transplanted patients at the Hospital de Clínicas of Unicamp to ensure better patient care and follow-up.

KEYWORDS: EBV, infection monitoring, hematopoietic stem cell transplantation

POST-TRANSPLANT OUTCOMES IN MYELODYSPLASTIC SYNDROME: A BRAZILIAN PRIVATE CENTER EXPERIENCE

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BACKGROUND:

Myelodysplastic syndrome (MDS) comprises a heterogeneous group of clonal hematopoietic disorders characterized by cytopenias and an inherent risk of transformation to acute myeloid leukemia (AML). Allogeneic hematopoietic stem cell transplantation (allo-HSCT) remains the only potentially curative therapy, although it is associated with substantial risks of relapse and non-relapse mortality (NRM). Pre-transplant risk stratification tools, such as the IPSS-M, guide prognostication and therapeutic decisions. Objective: To retrospectively evaluate post-transplant outcomes in patients with MDS undergoing allo-HSCT in a Brazilian private center.

METHODS:

We included all adult patients (>18 years) diagnosed with MDS and treated with allo-HSCT between December 2018 and January 2025. Data were retrospectively collected from electronic medical records. Outcomes analyzed included overall survival (OS), disease-free survival (DFS), cumulative incidence of relapse, NRM, causes of death, and post-relapse treatments. Survival analyses were stratified by IPSS-M risk categories.

RESULTS:

A total of 25 patients underwent allo-HSCT during the study period, with a median follow-up of 44.6 months. The 3-year OS was 73% (95% CI, 55–96%), and the median OS was not reached. The 3-year DFS was 51.8% (95% CI, 33–79%), with no median DFS reached. The 36-month cumulative incidence of relapse was 32% (95% CI, 11–52%), while NRM at the same time point was 16% (95% CI, 0–33%). Relapse occurred in 7 patients, with a median time to relapse of 3.78 months post-transplant. Post-relapse treatments included hypomethylating agents plus venetoclax (n=6) and donor lymphocyte infusion (DLI) (n=5). Five patients died, with causes including disease progression (n=2) and NRM (n=3). Among the cohort, 7 patients (28%) were classified as low or intermediate-low risk per IPSS-M, while 16 (64%) were in the intermediate-high to very-high risk categories; 2 patients were unclassifiable. Of the 7 relapsed patients, 5 (71%) were from the higher-risk IPSS-M strata at diagnosis.

CONCLUSION:

Allo-HSCT remains a potentially curative option for patients with MDS. However, relapse and NRM significantly affect long-term outcomes. The IPSS-M proved useful in stratifying post-transplant prognosis. Post-relapse management continues to be a major therapeutic challenge, underscoring the importance of optimized pre-emptive and maintenance strategies in this population.

KEYWORDS:

Myelodysplastic syndrome, Hematopoietic stem-cell transplantation (HSCT), Outcomes.

PREDICTORS OF INTESTINAL DOMINATION IN PATIENTS UNDERGOING ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

In patients undergoing allogeneic hematopoietic stem cell transplantation (allo-HSCT), a key feature of intestinal dysbiosis is intestinal domination. Intestinal domination, which is defined as a relative abundance of any single taxonomic unit of at least 30%, can occur in 28 to 65% of patients. Intestinal domination has also prognostic significance – it is associated with reduced overall survival, acute graft versus host disease severity, and bacteremia. Although intestinal domination is frequent and associated with poor outcomes, evidence on the predictors of intestinal domination remains limited.

OBJECTIVE:

In this study, we sought to identify predictors of intestinal domination in patients undergoing allo-HSCT.

METHODS:

This is an observational prospective cohort study of four hospitals. Subjects were patients >12 years old undergoing allo-HSCT. Fecal specimens were collected longitudinally at pre-determined time points (from prior to allo-HSCT to six months after). We performed 16S rRNA gene sequencing targeting the V3/V4 regions, and sequences were grouped into operational taxonomic units. Intestinal domination was defined as the presence of any single genus comprising at least 30% of the relative abundance within a given sample. Finally, we performed multivariate logistic regression models to identify patient-level predictors of intestinal domination.

RESULTS:

A total of 192 fecal specimens were collected from 69 patients. Among these specimens, 131 (68%) had intestinal domination. The top three dominant genera were: 1) Bacteroides (24%), 2) Akkermansia (10%), and 3) Phascolarctobacterium (8%). Multivariate regression models showed that no patient-level characteristic significantly predicted intestinal domination (see Table 1). Conclusion: Our findings suggest that no patient-level characteristic reliably predicts intestinal microbiota domination. Transient factors influencing the gut microbiota during allo-HSCT, such as antibiotic administration, may represent more robust and reliable predictors.

KEYWORDS: gastrointestinal microbiome; stem cell transplantation; prognosis.

PROFILE OF CHRONIC MYELOMONOCYTIC LEUKEMIA DIAGNOSIS OVER TEN YEARS IN A TERTIARY HOSPITAL IN NORTHEASTERN BRAZIL

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Chronic myelomonocytic leukemia (CMML) is a rare hematologic malignancy that combines features of myelodysplastic and myeloproliferative neoplasms. It predominantly affects elderly individuals and presents a heterogeneous clinical course. This retrospective study aimed to describe the clinical and therapeutic profile of patients diagnosed with CMML over the last ten years at a tertiary teaching hospital in Northeastern Brazil. A total of 13 cases were identified between 2013 and 2023, with a male predominance (69.2%) and a mean age at diagnosis of 57.2 years. Hematopoietic stem cell transplantation (HSCT) was performed in 4 patients (30.8%), including 3 men and 1 woman, with a mean age of 36.5 years at the time of transplantation. Among them, 75% received grafts from related donors. The average interval between diagnosis and HSCT was 13.5 months, and neutrophil engraftment occurred after a mean of 18 days. Hypomethylating agents were administered in 7 patients (53.8%), predominantly azacitidine, which aligns with literature data showing its use in up to 87.5% of CMML patients, particularly those at higher risk. Despite the central role of molecular mutations (such as ASXL1 and TET2) in current prognostic models like the CPSS-Mol or Mayo Molecular Model, molecular stratification was not performed in this cohort due to local limitations. These findings reflect a younger and more transplant-eligible population compared to international cohorts, as well as underutilization of molecular tools for risk assessment and hypomethylating drugs. The male predominance and age distribution were consistent with international cohorts. This study emphasizes the importance of early identification of transplant eligibility and access to donor search, as HSCT remains the only potentially curative treatment for high-risk CMML. Expanding access to molecular diagnostics and standardized prognostic models is essential to improve therapeutic decision-making in resource-limited settings. Further multicenter analyses are warranted to consolidate regional epidemiological patterns and therapeutic outcomes.

KEYWORDS: MYELOMONOCYTIC LEUKEMIA, ALLOTRANSPLANTION

Indicator	Investigator's Center	Literature
Total cases	13	-
Male (%)	69.2	70
Female (%)	30.8	30
Mean age at diagnosis	57.2	65
Mean age at transplantation	36.5	45
Transplanted patients (%)	30.8	20
Related donors (%)	75.0	70
Mean time until transplantation (months)	13.5	12-24
Mean time to engraftment (days)	18	14-21
Hypomethylating agent therapy (n, %)	7 patients (53.8%)	87.5% of patients used azacitidine

PROGRESSION FROM CLONAL HISTIOCYTOSIS TO ACUTE MYELOID LEUKEMIA: WHAT IS THE ROLE OF HEMATOPOIETIC STEM CELL TRANSPLANTATION IN THE TREATMENT APPROACH?

Maria Laura Chagas Fontoura Rocha[1], Emanelly Thays Muniz Figueiredo Silva Vasconcelos[1], Gustavo Mesquita de Oliveira[1], Felipe de Menezes Cunha[1], Beatriz Stela de Souza Pitombeira Araújo[1], Livia Andrade Gurgel[1], Karine Sampaio Nunes Barroso[1], Lucas Freire Castelo[1], Francisco Dário Rocha Filho, João Paulo Vasconcelos Leitão[1], Fernando Barroso Duarte[1]

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ABSTRACT:

Histiocytic and dendritic cell neoplasms are rare, representing less than 1% of soft tissue and lymph node malignancies. The 2008 WHO classification includes histiocytic sarcoma (HS), Langerhans cell sarcoma (LCS), Langerhans cell histiocytosis (LCH), follicular dendritic cell sarcoma (FDCS), interdigitating dendritic cell sarcoma (IDCS), and indeterminate dendritic cell tumors (IDCT). In Brazil, clinical and prognostic data are scarce.

NMO, 58- years old, reports daily fever, fatigue, syncope, and a 5 kg weight loss over six months. Referred to hematology with pancytopenia and circulating immature cells, she was initially suspected of having acute leukemia (AL). Bone marrow (BM) examination on October 7, 2024, showed erythroid dysplasia, increased macrophage activity, and negative immunophenotyping for AL. Biopsy revealed histiocytic infiltration with Langerhans-like giant cells, hemophagocytosis, and immunohistochemistry (IHC) positive for CD163 and glycophorin, suggesting clonal histiocytosis.

She received pulse corticosteroid therapy (dexamethasone) with no clinical improvement. On November 28, peripheral blood analysis detected blasts. A follow-up BM in December confirmed acute myeloid leukemia (AML) with 20% myeloid blasts. Geriatric assessment deemed the patient pre-frail and unfit for intensive chemotherapy or hematopoietic stem cell transplantation (HSCT). Treatment with low-dose cytarabine and venetoclax was initiated. After one cycle, blast count was partially reduced; however, the second cycle revealed hypocellular marrow, grade II myelofibrosis, and extensive fibrosis on IHC.

The patient developed multiple severe infections requiring broad-spectrum antibiotics. Due to clinical decline, care was transitioned to palliation. She died five months after diagnosis.

Histiocytoses involve pathologic accumulation of macrophages or dendritic cells in tissues, triggering inflammation and organ dysfunction. LCH lesions

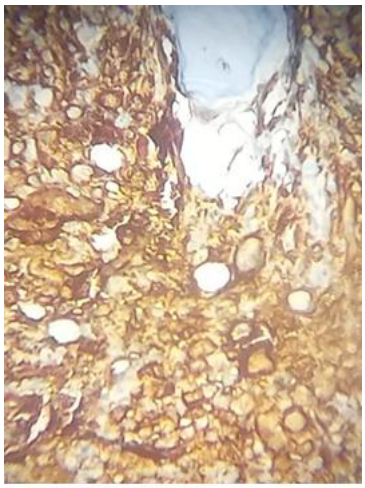
include CD1a+ and CD207+ cells within an inflammatory milieu of macrophages, lymphocytes, eosinophils, and multinucleated giant cells. Clinical presentations are site-dependent, affecting skin, liver, spleen, or bone marrow, and may include cytopenias and cutaneous lesions.

Hemophagocytic lymphohistiocytosis (HLH) is a life-threatening hyperinflammatory syndrome marked by fever, splenomegaly, and elevated ferritin and soluble IL-2 receptor alpha (sIL2Rα). It can be primary (genetic) or secondary to infection or malignancy. Chemotherapy can reduce HLH symptoms in malignancy-associated cases.

Standard HLH treatment follows the HLH-94 protocol (dexamethasone + etoposide). For LCH, single-agent cytarabine or cladribine is preferred over combination regimens. Allogeneic HSCT is reserved for refractory or genetically defined cases, though reduced-intensity conditioning remains challenging due to graft failure risk. Molecularly targeted therapies represent a promising frontier. HSCT is potentially curative for refractory LCH, yet the ideal conditioning approach remains under debate

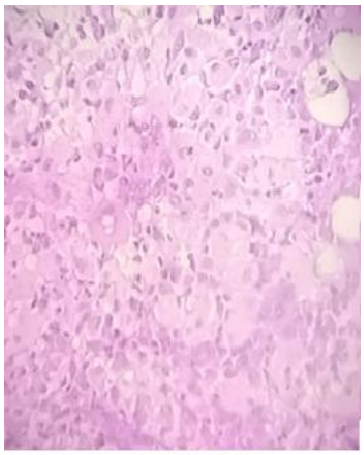
KEYWORDS:

Clonal histiocytosis, Acute myeloid leukemia, Hemophagocytic lymphohistiocytosis, Hematopoietic stem cell transplantation, Hematology



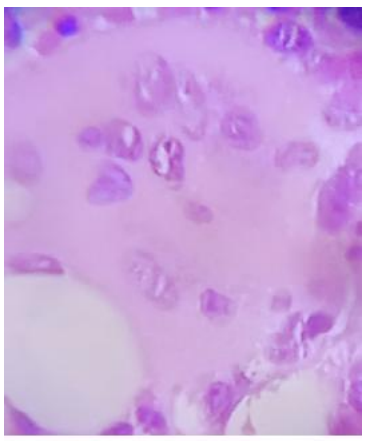
1 – Immunohistochemistry

CD163 LABELING. STRONG, DIFFUSE 200X



2 - H.E. Bone marrow 200x.

Several histiocytes with clear cytoplasm.



3 - multinucleated giant cell HE 400x bone marrow

RECENT ADVANCES IN GENE THERAPY FOR SICKLE CELL DISEASE: A SCOPING REVIEW

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INTRODUCTION:

Sickle Cell Disease (SCD) is a common hereditary hemoglobinopathy characterized by chronic hemolytic anemia, vaso-occlusive crises (VOCs), and progressive organ damage. While allogeneic hematopoietic stem cell transplantation (allo-HSCT) is the only established curative option, its application is limited by the need for matched donors. In this context, autologous hematopoietic stem cell (HSC) gene therapy has emerged as a promising alternative to overcome this limitation, offering curative potential to a larger patient population.

OBJECTIVE:

To list the most recently developed gene therapy modalities for the treatment of sickle cell disease.

METHOD:

Scoping review based on a bibliographic search in the MEDLINE database, using the terms "Sickle cell" and "Gene editing". Seven articles were selected from the results of papers that were not reviews and that included free full text published between 2020 and May 2025.

DISCUSSION:

The studies analyzed demonstrate significant advances for gene therapy in autologous hematopoietic stem cell transplantation for SCD. A gene-editing approach mediated by CRISPR/Cas9, wich aimed at reactivating fetal hemoglobin (HbF) expression through the editing of the BCL11A gene in CD34+ hematopoietic stem and progenitor cells, has shown promising results, eliminating vaso-occlusive crises in 97% of patients for a period of 12 months or more. Furthermore, a Zinc Finger Nuclease (ZFN)-Mediated disruption of the BCL11A led to a 3-fold increase in HbF levels, with biallelic edited cells reaching 50% HbF compared to 22% in unedited cells. This editing also resulted in reduced sickling under hypoxic conditions. Another strategy involves CRISPR-Cas9 combined with recombinant adeno-associated virus vectors (rAAV6) to express an anti-sickling variant of β-globin in hematopoietic stem cells, resulting in 60% allelic correction of the HBB gene in CD34+ cells in vitro. Patients treated with these therapies have shown improvements in quality of life and a reduction in transfusion dependence. Despite the curative potential, challenges such as long-term safety, optimization of gene delivery, and high costs still need to be addressed.

CONCLUSION:

Gene-editing-based strategies, such as HbF reactivation and β-globin gene correction, represent a paradigm shift in the treatment of SCD. Current clinical outcomes are robust, demonstrating efficacy in reversing the disease phenotype and significantly improving patients' quality of life. Although long-term safety and therapeutic accessibility remain significant hurdles, ongoing advances solidify gene therapy as one of the most promising pathways toward a widely applicable cure for sickle cell disease.

KEYWORDS: Sickle Cell Disease, Gene editing, CRISPR/Cas9, fetal hemoglobin.

SEVERE SPLENOMEGALY IN PRIMARY MYELOFIBROSIS RESISTANT TO TYROSINE KINASE INHIBITORS: CASE REPORT

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2 - D'Or Oncology, Brasilia - DF, Brazil.

INTRODUCTION:

Myelofibrosis is a chronic myeloproliferative neoplasm characterized by bone marrow fibrosis, extramedullary hematopoiesis (causing splenomegaly), and risk of leukemic transformation. Diagnosis relies on bone marrow biopsy findings (megakaryocytic atypia, fibrosis) and driver mutations (JAK2, CALR, MPL). Treatment involves JAK inhibitors, such as ruxolitinib, and when possible, allogeneic hematopoietic stem-cell transplantation (HCT) as the only curative option.

OBJECTIVE: To present a clinical case of PMF unresponsive to JAK inhibitor.

METHOD: Case report and scope review.

CASE REPORT:

A 58-year-old man with hypertension, under angiotensin II receptor antagonist, presented with splenomegaly, without B symptoms. Blood counts were normal, with elevated LDH. Immunophenotyping was normal. Bone marrow biopsy (May 2024) showed panhypercellularity, granulocytic and erythroid maturation, megakaryocytic atypia, reticulin and collagen fibrosis — confirming Primary Myelofibrosis (MF-1). He was unresponsive to ruxolitinib and erythropoietin. A haploidentical related allogeneic HCT was performed on August 29, 2024, using reduced intensity conditioning (RIC) and GVHD prophylaxis with cyclophosphamide, tacrolimus, and mycophenolate mofetil. Neutrophil and platelet engraftments occurred on September 14 and 18, 2024, respectively. Post-transplant complications included veno-occlusive disease, febrile neutropenia, hepatic GVHD, and carbapenem-resistant Klebsiella oxytoca colonization.

DISCUSSION:

Although splenomegaly predominated, common PMF features like anemia, hepatomegaly, cachexia, and bone pain were absent. JAK inhibitors are first-line for symptom and spleen control but have limited effect on anemia, often requiring erythropoietin. These agents are frequently used pre-HCT. In refractory cases, HCT remains the only curative strategy, though associated with high morbidity. Conditioning regimens (MAC vs RIC) offer comparable long-term outcomes, with RIC preferred in older patients. New agents like momelotinib, fedratinib, and alternative JAK inhibitors are under investigation to improve pre-HCT disease control.

CONCLUSION:

This report describes a patient with MF-1, diagnosed in May 2024, presenting with isolated splenomegaly, unresponsive to ruxolitinib and erythropoietin, progressing to haploidentical HCT with RIC. Post-transplant, the patient developed severe complications, illustrating the risks associated with this curative approach. The case reinforces the urgent need for new therapies for JAK inhibitor-refractory PMF and strategies to reduce HCT-related morbidity and improve survival.

KEYWORDS: Primary Myelofibrosis; JAK Inhibitor Refractoriness; Allogeneic Hematopoietic Stem-Cell Transplantation; Reduced Intensity Conditioning; Graft-versus-Host Disease.

THE JOURNEY OF PATIENTS WITH MYELODYSPLASTIC SYNDROME AND ACUTE MYELOID LEUKEMIA TREATED WITH A BCL-2 INHIBITOR UNTIL ALLOGENEIC TRANSPLANTATION

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INTRODUCTION:

Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is the only potentially curative strategy for high-risk myelodysplastic syndrome (HR MDS) and for many patients with acute myeloid leukemia (AML). However, eligibility is often limited by advanced age, comorbidities, and response to initial treatment. The introduction of the BCL-2 inhibitor venetoclax, in combination with hypomethylating agents or low-dose cytarabine, as demonstrated in the VIALE-A and VIALE-C trials, has expanded the available therapeutic options. Nevertheless, data in real-world settings remain scarce.

OBJECTIVE:

To evaluate the clinical profile, therapeutic response, and feasibility of allo-HSCT in patients with HR MDS and AML treated with venetoclax-based regimens.

POPULATION:

Twenty-four patients diagnosed with HR MDS or AML were included. They were treated at a tertiary care institution located in Fortaleza, Brazil, between 2019 and 2024.

METHODS:

This was a retrospective observational study with descriptive analysis of clinical, laboratory, cytogenetic, and molecular variables. Patients were treated with either azacitidine plus venetoclax (n=11) or low-dose cytarabine plus venetoclax (n=13). One patient initially received cytarabine and switched to azacitidine by the third cycle. Treatment response was assessed according to the ELN criteria for AML and the IWG criteria for MDS.

RESULTS:

Among the 24 patients, 15 were female and 9 male, aged between 37 and 81 years (median: 65 years). Six patients had HR MDS, including four with excess blasts, with a median IPSS-R score of 6. Eighteen patients were diagnosed with AML, 11 of which were secondary AML—mainly evolving from MDS, as well as cases associated with CMML, primary myelofibrosis, and polycythemia vera. The number of treatment cycles ranged from 1 to 10, with a median of 2.5 cycles. Seven patients (29.1%) achieved complete remission (CR), with a median of one cycle to response. The longest time to response occurred in a patient with MDS-EB2 (4 cycles). Among the 18 AML cases, six achieved CR, but none underwent transplantation, mainly due to clinical frailty (4 cases) or early relapse (2 cases). Of the six MDS patients, two underwent allo-HSCT—one in CR and the other without a significant reduction in blast percentage; the remaining patients were deemed ineligible due to frailty. The median overall survival of the cohort was 330 days, and it was significantly longer in patients with MDS (1129 days) compared to those with AML (304 days; $p = 0.039$).

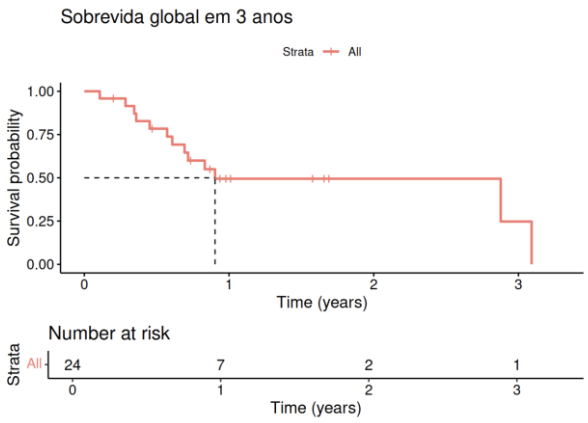
CONCLUSION:

The combination of venetoclax with hypomethylating agents or low-dose cytarabine showed significant response rates in patients with HR MDS and AML treated in real-world settings, including those with secondary disease. However, clinical frailty and early relapse remain major limitations to the performance of allo-HSCT, highlighting the need for earlier interventions and personalized strategies to expand transplant eligibility.

KEYWORDS:

BCL-2 Inhibitor, Acute Myeloid Leukemia, Myelodysplastic Syndrome

1. c3-year overall survival curve

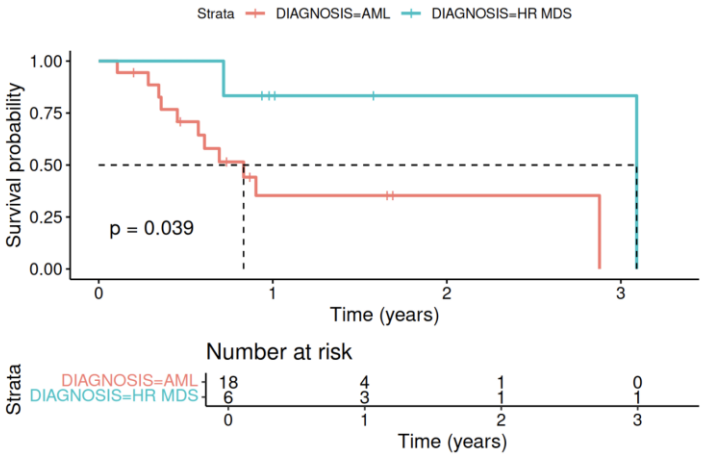


2. Cohort overall survival data

Characteristic	1y	2y	3y
Overall	49% (32%, 76%)	49% (32%, 76%)	25% (5.8%, 100%)

Characteristic	Median survival
Overall	330 days

3. 3-year overall survival curve by diagnosis



4. Overall survival data by diagnosis

Characteristic	1y	2y	3y
DIAGNOSIS			
AML	35% (17%, 72%)	35% (17%, 72%)	— (—, —)
HR MDS	83% (58%, 100%)	83% (58%, 100%)	83% (58%, 100%)

Characteristic	Median survival
DIAGNOSIS	
AML	304 days

Characteristic	Median survival
HR MDS	1,129 days

THE PROGNOSTIC SIGNIFICANCE OF INTESTINAL DOMINATION IN ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION VARIES ACCORDING TO THE DOMINANT GENERA AND POPULATION BEING EVALUATED

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INTRODUCTION:

In patients undergoing allogeneic hematopoietic stem cell transplantation (allo-HSCT), an important prognostic factor is the expansion of a single microbiota genus leading to intestinal domination. In international studies, intestinal domination was predominantly caused by the expansion of Enterococcus, which was associated with a decrease in overall survival and an increase in graft versus host disease (GvHD) severity. Nevertheless, preliminary data from on ongoing multi-center study demonstrated that Enterococcus domination is a rare event in Brazilian patients. In Brazilian patients, intestinal domination events are primarily driven by Bacteroides, Akkermansia, Phascolarctobacterium, and Escherichia-Shigella. Whether domination by these genera carries prognostic significance remains unclear.

OBJECTIVE:

In this study, using a cohort of Brazilian patients undergoing allo-HSCT, we sought to evaluate the impact of intestinal domination by the four aforementioned genera on overall survival and the cumulative incidence of GvHD.

METHODS:

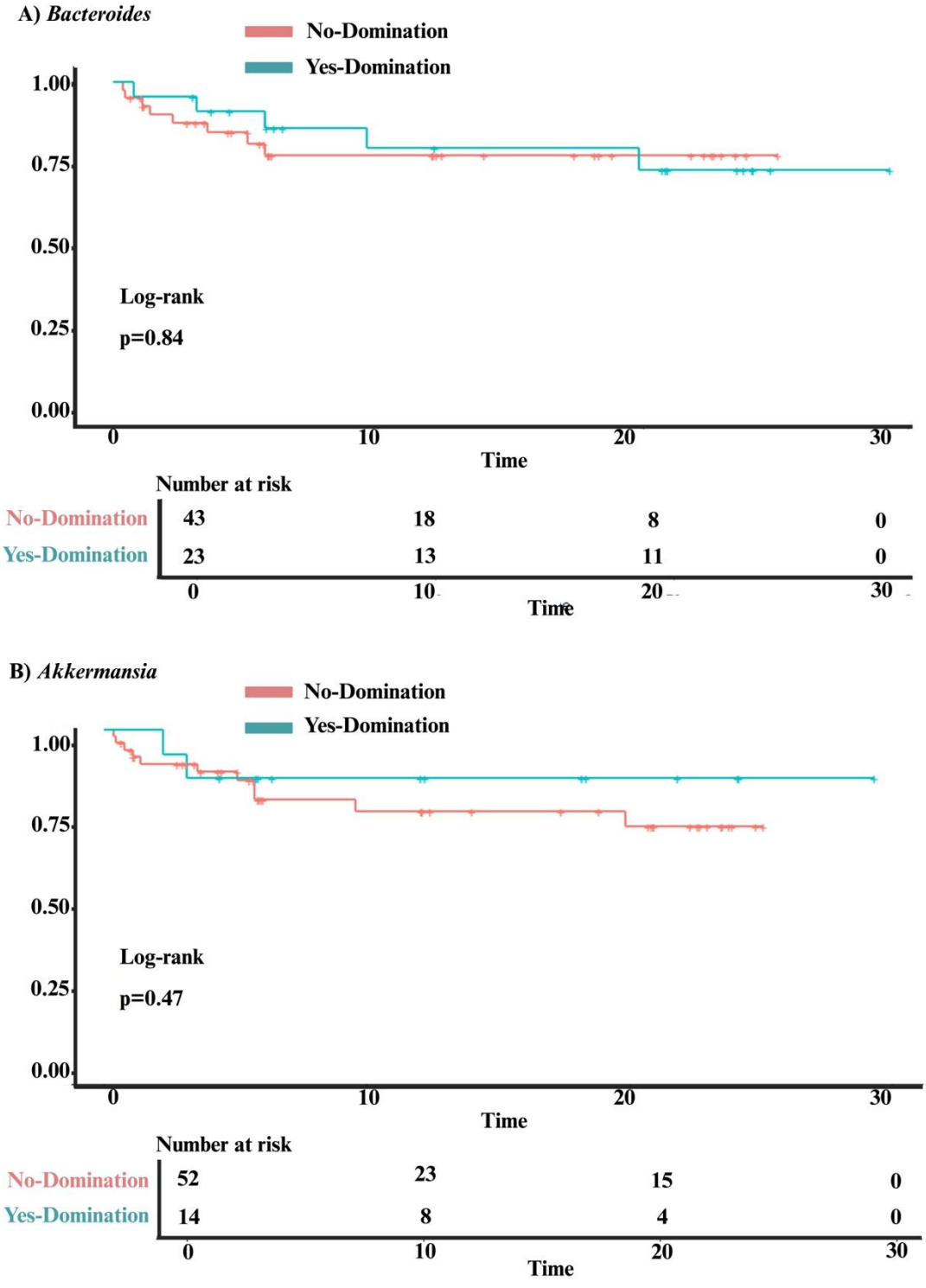
This is a multicenter, observational prospective study, approved by the Research Ethical Committee. Subjects were patients >12 years old undergoing allo-HSCT. Fecal specimens were collected longitudinally at pre-determined time points (from prior to allo-HSCT to six months after). Fecal DNA was extracted and 16S sequencing was performed by using Illumina platform. Bioinformatic analysis was performed, and the operational taxonomic units were used to determine the intestinal domination. Overall survival was analyzed using the Kaplan-Meier methodology and survival curves were compared using the log-rank test. A Cox regression analysis was used to evaluate the association between intestinal domination and the cumulative incidence of GvHD. Results: During the study period, 69 patients provided 192 fecal specimens. Our analysis identified that intestinal domination by these four genera is not significantly associated with overall survival (see Figure 1). Although univariate analysis revealed a significant association between Phascolarctobacterium domination and cumulative incidence of aGvHD (HR 2.39; 95% CI 1.08-5.31; p=0.032), this was not significant in a multivariable model after adjusting for age, sex, underlying diagnosis, conditioning regimen, donor sex, stem cell source, and donor type (HR 1.75; 95% CI 0.73-4.20; p=0.2). For the other three genera, no significant associations with GvHD were found.

CONCLUSION:

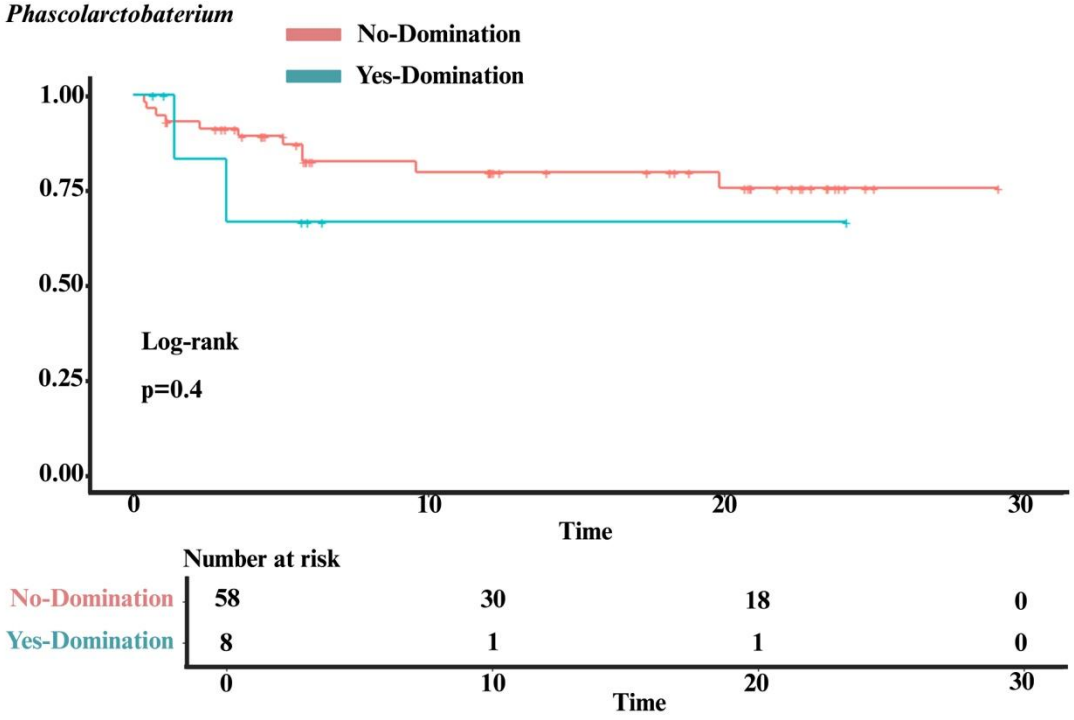
Our findings suggest that the prognostic significance of intestinal domination may vary according to the dominant genus and the cohort being evaluated. Future studies are desired to clarify the dominant genera that are more likely to have prognostic significance.

KEYWORDS: gastrointestinal microbiome; stem cell transplantation; prognosis

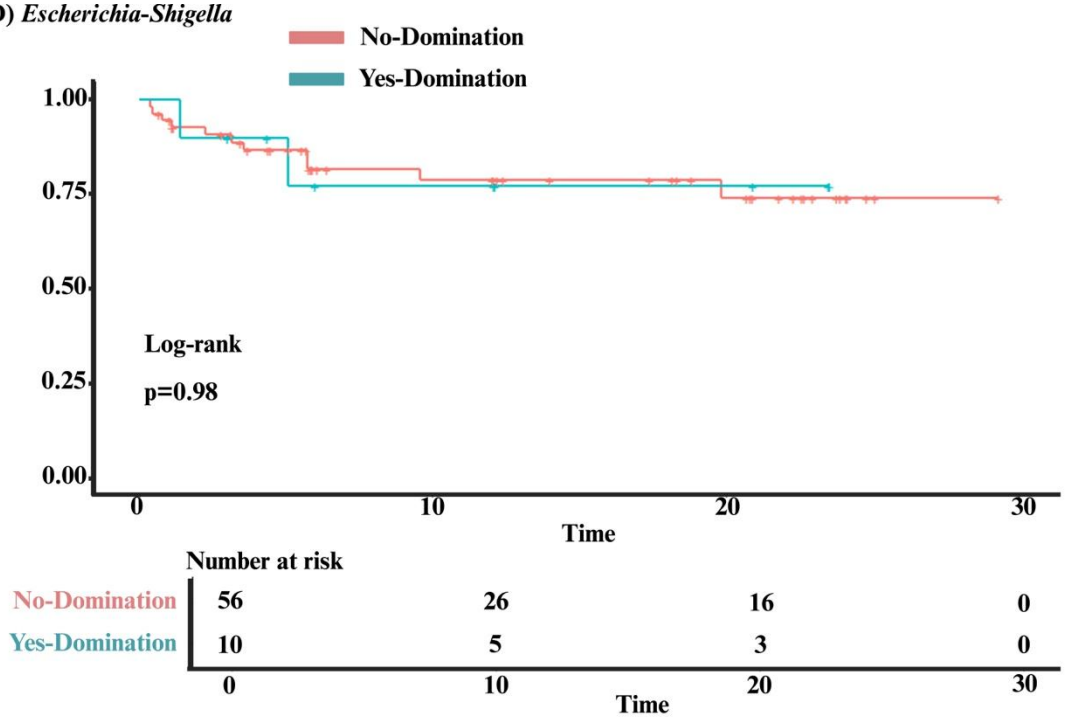
FIGURE 1. **The impact of intestinal domination on overall survival. A) Bacteroides. B) Akkermansia. C) Phascolarctobaterium. D) Escherichia-Shigella.**



C) *Phascolarctobaterium*



D) *Escherichia-Shigella*



VALIDATION OF HUMAN LEUKOCYTE ANTIGEN TYPING BY NANOPORE SEQUENCING: COMPARISON WITH THE SSO TECHNIQUE AND APPLICATION IN ROUTINE DIAGNOSTICS.

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INTRODUCTION:

Ensuring compatibility between donor and recipient human leukocyte antigen (HLA) systems is crucial for minimizing the risk of acute and chronic rejection during transplantation. The Sequence-Specific Oligonucleotide (SSO) technique is a well-established method utilized in specialized laboratories for typing various HLA loci, including HLA-A, HLA-B, HLA-C, HLA-DR, HLA-DQ, and HLA-DP, which aids in identifying potential matches. Recently, nanopore sequencing technology, developed by Oxford Nanopore Technologies, has revolutionized high-resolution genotyping approaches, particularly in HLA typing. This technology's primary advantage lies in its ability to sequence long PCR-amplified DNA fragments—spanning thousands of base pairs—which allows for the resolution of allelic ambiguities that are often encountered with conventional methods.

OBJECTIVE:

This study evaluated the performance of a nanopore sequencing-based HLA typing approach developed by Omixon Biocomputing Ltd., comparing it with the traditional SSO technique and its application in routine laboratory diagnostics. Study Population: Eleven peripheral blood samples from patients referred to the laboratory for HLA typing were analyzed.

METHODS:

Genomic DNA was extracted using the Biopur Mini Spin Plus kit. SSO typing was conducted using LABType kits for HLA-A, HLA-B, HLA-DRB1, HLA-DQA1 and HLA-DQB1, all provided by One Lambda. For the sequencing-based approach, the NanoTYPE™ HLA kit (Omixon) was used, with locus-specific amplification performed on a Veriti thermocycler (Thermo Fisher). Amplified products were quantified using the Qubit BR assay, and 200ng of each sample was barcoded for sequencing on an R9.4.1 flow cell (FLO-MIN106D) utilizing the MinION® device. Data acquisition and analysis were conducted using MinKNOW® software. The concordance between the methodologies was assessed at a two-field resolution, with graphical analyses performed using GraphPad software.

RESULTS:

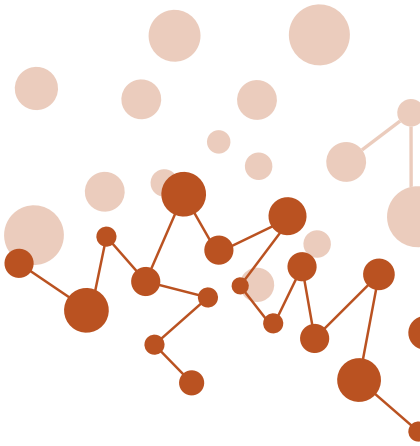
The findings demonstrated 100% concordance between the two methodologies across all examined loci, with no discrepancies identified. No alleles detected by nanopore sequencing were found to diverge from the ambiguities listed in the SSO panel. Furthermore, SSO data served as a reference to validate the MinION®, which facilitated the establishment of acceptance criteria for its integration into the laboratory's diagnostic workflow.

CONCLUSIONS:

This study confirms the technical feasibility and reliability of nanopore sequencing for HLA typing in a routine laboratory environment, showcasing its high accuracy. Additionally, this technology presents several operational advantages, including lower infrastructure requirements and reduced initial costs compared to the SSO method. With the growing adoption of this approach in HLA typing laboratories, a gradual reduction in costs is anticipated, which will promote its wider application in clinical practice.

KEYWORDS: Nanotype, SSO, HLA.

AUTOLOGOUS HCT



AUTOLOGOUS STEM CELL TRANSPLANTATION VERSUS CAR-T CELL THERAPY IN FIRST RELAPSE OF B-CELL LYMPHOMAS: COMPARISON OF COMPLETE RESPONSE RATES

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INTRODUCTION:

Diffuse large B-cell lymphoma (DLBCL) is the most common form of aggressive non-Hodgkin lymphoma. A significant proportion of patients with DLBCL experience relapse or refractory disease, requiring subsequent therapeutic approaches. The management of the first relapse has traditionally involved salvage chemotherapy followed by autologous hematopoietic stem cell transplantation (ASCT). However, the emergence of CAR-T cell therapies has introduced a potentially more effective alternative for patients with early relapse or chemotherapy-refractory disease.

OBJECTIVE:

To compare the complete response (CR) rates of CAR-T cell therapy versus ASCT in the management of first relapse in DLBCL, based on current literature.

METHODS:

[A narrative literature review was conducted, including studies published in English in the last ten years from the PubMed, ScienceDirect, and OpenEvidence databases. The search terms used were: "Diffuse Large B-Cell Lymphoma," "Autologous Stem Cell Transplantation," "CAR-T Cells," "First Relapse," "Axicabtagene," "Lisocabtagene," and "Tisagenlecleucel." Clinical studies and systematic reviews were considered eligible for inclusion.

RESULTS:

Eight studies were selected for in-depth analysis, including the four principal randomized clinical trials in this area. Three out of 4 studies provided evidence supporting the potential of CAR-T cell therapy as an alternative to ASCT already as second-line treatment, especially for patients with early relapse (<12 months) or refractory disease. In the ZUMA-7[1] study (n=1,297), Axi-cel showed an impressive 56% CR rate - substantially higher than what has been historically seen with standard salvage chemotherapy followed by ASCT (typically 20–30% CR in this setting). The TRANSFORM[2] study compared Lisocabtagene Maraleucel with ASCT (n=184) within 12 months of first-line treatment failure, finding that the CAR-T arm had a higher CRR (66%) compared to ASCT (39%). In the TRANSCEND[3] study (n=83), liso-cel achieved an overall response rate of 73%, with 53% of patients experiencing CR, despite the inclusion of a challenging patient population—69% with refractory disease and 33% with prior ASCT. Conversely, the BELINDA4 study (n= 236) showed no statistical benefit of tisagenlecleucel (CAR-T anti-CD19) over standard of care with identical CR rate (28%). This could be due to differences in population, CAR-T cell type or other confounding factors. Nonetheless, some real-world observations support the superiority of CAR-T in early relapse. A multicenter study5 involving 318 patients showed a 12-month overall survival of 63% with CAR-T versus 48% with ASCT.

CONCLUSIONS:

Some studies showed good potential for CAR-T in relapsed DLBCL. Yet, inconsistent findings—such as those from the BELINDA trial—highlight the need for further research to elucidate factors affecting outcomes, such as lymphoma genotype and CAR-T target, and to optimize patient selection and treatment strategies.

BEAM VS. LEAM CONDITIONING REGIMENS FOR AUTOLOGOUS STEM CELL TRANSPLANTATION IN HODGKIN'S LYMPHOMA: A RETROSPECTIVE ANALYSIS

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INTRODUCTION:

High-dose chemotherapy (HDT) followed by autologous hematopoietic stem-cell transplantation (ASCT) is widely used in patients with lymphoproliferative diseases. BEAM (BCNU/carmustine, etoposide, cytarabine, and melphalan) is the most frequently used conditioning regimen in Europe for relapsed/refractory lymphomas, whereas LEAM (lomustine, etoposide, cytarabine, and melphalan) has become a practical alternative when carmustine is unavailable.

OBJECTIVE:

To compare the BEAM versus LEAM conditioning regimens in Hodgkin's disease regarding hematologic toxicity, neutrophil engraftment time, infectious complications (mucositis, colitis, bloodstream infection), need for parenteral nutrition, and post-transplant PET-CT response.

METHODS:

Retrospective study of 31 patients (15–64 years) with relapsed/refractory Hodgkin lymphoma. Conditioning distribution: BEAM = 13, LEAM = 18. Data was collected from a single bone-marrow-transplant center between October 2020 and May 2025.

RESULTS:

All patients developed severe neutropenia and thrombocytopenia; mean time to neutrophil engraftment was 11.75 days with BEAM and 11.5 days with LEAM. Comparing BEAM versus LEAM, rates of mucositis were 23 % vs 44 %, colitis 53 % vs 83 %, and bloodstream infection 23 % vs 11 %. Parenteral nutrition was required in 18 % (BEAM) and 22 % (LEAM). Mean hospitalization was 21.4 days (BEAM) versus 22.44 days (LEAM). Among the patients evaluated by PET three months post- transplant, 10/11 (91 %) in the BEAM group and 11/12 (92 %) in the LEAM group achieved complete response. At diagnosis, 84 % of BEAM and 72 % of LEAM patients were stage III/IV; 61 % (BEAM) and 50 % (LEAM) had received more than two prior lines of chemotherapy.

CONCLUSION:

BEAM and LEAM appear to deliver comparable clinical efficacy, with high complete-response rates on PET-CT three months after transplant (91 % vs 92 %). In settings where BCNU is scarce, lomustine-based regimens represent suitable alternatives without apparent increases in toxicity or loss of effectiveness. Selection can therefore be guided by drug availability, institutional experience, and patient comorbidities.

KEYWORDS: Hodgkin´s lymphoma. Autologous stem cell transplantation. Conditioning chemotherapy.

CELL PROCESSING CENTER: PRODUCTION AND CLINICAL APPLICATIONS

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1. Institutional affiliation: 1.Centro de Hematologia e Hemoterapia do Estado do Ceará

INTRODUCTION:

The Cell Processing Center (CPC) is responsible for processing and cryopreserving hematopoietic progenitor cells (HPC) from 5 transplant centers in the state, four of which are private and one public. As of December 2022, the cryopreservation technique based on cell concentration was adopted. We currently use a concentration of 300,000 cells/mm^3. This change was implemented based on scientific evidence showing that cryopreservation by cellularity significantly reduces the risk of complications during infusion, especially neurological events. In addition, this method contributes to less cell loss after the thawing process, ensuring greater viability and efficacy of cryopreserved CPH. AIM: To present and analyze the production of a Cell Processing Center (CPC), with emphasis on the safe and efficient handling of cells for therapeutic use.

MATERIALS AND METHODS:

A retrospective analysis was carried out of the laboratory records of patients seen by the CPC between January 2023 and April 2025. The study included autologous patients undergoing cell mobilization with the aim of collection by apheresis and subsequent cryopreservation for use in hematopoietic progenitor cell transplantation (HPCT). The following clinical parameters were collected and analyzed: Number of apheresis performed per patient; Age; Diagnosis; Number of cryopreserved bags; Cell viability after processing and grafting of patients transplanted in the period analyzed.

RESULTS:

A total of 258 autologous patients from 5 transplant centers were treated during the period under evaluation, 144 (55.8%) from the public network and 114 (44.2%) from the private network. 306 CPH bags were collected and 799 bags of up to 100 mL were cryopreserved (an average of 3 bags per patient). 48 patients underwent two apheresis procedures. The average age of the patients was 52, ranging from 19 to 76. The main indications for autologous BMT were Multiple Myeloma (60.4%), Hodgkin's Lymphoma (19%), Non-Hodgkin's Lymphoma (16.3%) and others (4.3%). The average post-processing cell viability was 98.5%. Of the transplants carried out during the period, the average neutrophil engraftment time was 10 days, ranging from D+7 to D+17.

CONCLUSION:

The data presented demonstrates the effectiveness of the CPH cryopreservation service in caring for 258 autologous patients from different transplant centers. There was a predominance of indications related to Multiple Myeloma, followed by Lymphomas. The average post-processing cell viability was 98.5%, demonstrating the quality of the processed material. The average time for neutrophil grafting was (10 days). These results reinforce the importance of standardizing processes, guaranteeing safety and efficacy in TCPH.

KEYWORDS: Hematopoietic Progenitor Cells (HPC); hematopoietic progenitor cell transplantation HPTC; Cryopreservation by cellularity

ENSURING GOOD PRACTICES AND RISK MANAGEMENT IN THE TRANSFER OF CRYOPRESERVED CELL UNITS TO A NEW CELL PROCESSING CENTER: A SAFE MODUS OPERANDI

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INTRODUCTION:

The transportation of hematopoietic stem cells (HSCs) requires meticulous planning, strict adherence to good laboratory practices, and precision in execution, making it a determining factor in the therapeutic success of HSCT. To ensure cell viability and minimize risks, the process must follow rigorous protocols, including continuous temperature control, defined transport times, appropriate packaging, and full traceability of the cellular product. The objective of this study is to present a detailed plan for the safe and efficient transfer of cryopreserved cell units to a new Cell Processing Center (CPC).

METHODS:

For methodological purposes, the approach was structured into three main phases: risk mapping, strategic planning, and operational execution.

RISK MAPPING:

The table below outlines the risk mapping along with the corresponding mitigation measures and barriers.

RISK MAPPING RELATED TO THE TRANSFER OF CRYOPRESERVED CELL UNITS	
Risk	**Barrier/Treatment**
Team lacking process knowledge	Team training
Occupational accidents	Biosafety training and use of appropriate PPE
Outsourcing of transportation	Hiring a licensed provider
Packaging adequacy	Provision of appropriate and validated tertiary packaging
Temperature increase	Provision of large quantity of cryopreservative material, continuous monitoring, and pilot-tested validation
Unit rupture	Use of thermal blankets and cushions to reduce impact between units
Undocumented processes	Development of planning, shipping and receiving documentation, and transport authorization
Inadequate cold chain equipment	Installation, operational, and thermal qualification of cryopreservation equipment and up-to-date maintenance
Inadequate infrastructure at destination institution	Qualification of storage room and alignment with maintenance/clinical engineering team and management
Loss of cell quality and compromise of BMT	Storage monitoring and stability plan.

Abbreviations: Bone Marrow Transplant (BMT); Personal Protective Equipment (PPE).

TRANSFER PLANNING:

The transfer of cryopreserved cell units was preceded by an initial planning phase, structured into three key stages: 1) Detailed assessment of the materials stored at the originating institution, including quantification of units and aliquots, cell types, cryopreservation history, and current storage locations. 2) Evaluation of storage conditions at the new CPC facility, including assessment of the storage room infrastructure in accordance with the requirements established by RDC 836/2023. 3) Coordination of all personnel involved in the process.

EXECUTION OF THE CELL UNIT TRANSFER:

The first day of transfer was considered a pilot phase to assess key variables relevant to the execution of the process, such as travel time, optimal route, environmental conditions, and the most suitable time of day. It was also essential to determine the safe and adequate time required for transferring the cells into the new cryopreservation equipment.

RESULTS:

The transfer of cryopreserved cell units was carried out according to the established plan, strictly following the steps previously described. A total of 425 units were transferred, including 277 units originally cryopreserved in mechanical freezers (-80°C) and 148 units previously cryopreserved in liquid nitrogen (< -150°C).

To complete the transfer of all units, 14 road transport trips were made over the course of 7 days. During the process, continuous temperature monitoring indicated stable conditions within ideal parameters, with no significant deviations that could compromise cell viability. No incidents related to equipment failure, transport issues, or cell unit ruptures were reported.

CONCLUSION:

The implementation of detailed risk management strategies and a well-structured transfer plan proved effective, as there were no losses or damage to the cell units. Team training and coordination were essential to the success of the operation.

This work demonstrates that the adoption of preventive strategies and strict process control throughout every stage of the transfer is essential to minimize risks and preserve the quality of cryopreserved cells. It serves as a valuable reference for future relocations in similar centers.

EVALUATION OF CYTOGENETIC BIOMARKERS FOR RISK STRATIFICATION IN MULTIPLE MYELOMA PATIENTS ELIGIBLE FOR BONE MARROW TRANSPLANTATION IN A NORTHEASTERN BRAZILIAN STATE

Maria Luiza Rocha da Rosa Borges[1]; Raysa Samanta Moraes Laranjeira[1]; Felipe de Menezes Cunha[2]; João Paulo de Vasconcelos Leitão[2]; Karine Sampaio Nunes Barroso[2]; Livia Andrade Gurgel[2]; Lucas Freire Castelo[2]; Beatriz Stela Gomes de Souza Pitombeira Araujo[2]; Jacqueline Holanda de Souza[2], Sérgio Luiz Arruda Parente Filho[2]; Fernando Barroso Duarte[1,2].

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Multiple myeloma (MM) is a malignant hematologic neoplasm characterized by the deregulated clonal proliferation of plasma cells in the bone marrow, leading to the abnormal production and secretion of monoclonal immunoglobulin. MM is estimated to account for approximately 1% of all cancers. Among risk markers, cytogenetic abnormalities are predictive of diagnosis and prognosis in MM.This study aimed to evaluate the presence of key cytogenetic biomarkers using conventional cytogenetics and fluorescence in situ hybridization (FISH) targeting t(4;14)(p16;q32), t(11;14)(q13;q32), and del(17p) in treated MM patients eligible for bone marrow transplantation in a northeastern Brazilian state. Between Feb/24 and May/25, 30 patients were analyzed 56% female and 44% male with a mean age of 68 years. Conventional cytogenetic analysis was performed in 13 patients, five of whom (38.5%) presented abnormal karyotypes. FISH was performed in all patients and identified TP53 deletion in five patients (17%), gain of IGH gene signals in one patient (3.3%), and FGFR3 gene amplification in one patient (3.3%), associated with a karyotype showing t(8;22)(q24;q21).Analysis of clinical outcomes in relation to cytogenetic alterations revealed that all five patients with FISH abnormalities were eligible for transplant: one underwent transplant and is currently at D+365 with VGPR status; one underwent two transplants and experienced disease progression; one was contraindicated for autologous transplant due to chronic liver disease, had disease progression, initiated daratumumab therapy, but was lost to follow-up; and two are awaiting transplant scheduling.Patients with abnormal karyotypes but normal FISH results were diagnosed with monoclonal gammopathy of undetermined significance and were not transplant candidates. One patient presented both karyotypic and FISH abnormalities, is transplant-eligible, and is awaiting consultation for pre-conditioning.Additionally, six patients with normal karyotype and FISH underwent transplantation: two achieved complete response (CR) by D+180, three showed very good partial response (VGPR) by D+180, and one experienced post-autologous transplant relapse and remains on lenalidomide therapy. Del(17p) (TP53) occurs in approximately 20% of MM cases and is considered a high-risk secondary cytogenetic marker, associated with poor prognosis. Alterations involving the IGH gene are observed in about 55% of cases, primarily via translocations, with isolated gene amplifications being less frequent. The FGFR3 gene, when involved in the t(4;14)(p16;q32) translocation, leads to gene overexpression, contributing to resistance to standard therapies. Isolated amplification of FGFR3 is rare and its prognostic significance in MM remains unclear.Therefore, identifying cytogenetic biomarkers at diagnosis and during pre-transplant assessment is critical for accurate risk stratification and effective therapeutic decision-making.

KEYWORDS: ultiple Myeloma, Risk Stratification, Cytogenetics

EVALUATION OF POST-TRANSPLANT OUTCOMES AND THE TIME BETWEEN THE ADMINISTRATION OF MELPHALAN AND THE INFUSION OF AUTOLOGOUS PROGENITOR CELLS IN PATIENTS WITH MULTIPLE MYELOMA

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INTRODUCTION:

Autologous stem cell transplant (ASCT) is an important part of the treatment of multiple myeloma. Patients must undergo a conditioning regimen, which traditionally uses high-dose melphalan. However, the timing of melphalan administration in relation to the infusion of stem cells (TimeMel) is not clearly defined. A 24-hour waiting period, or even up to 48 hours, has been implemented by many institutions due to melphalan's potential cytotoxic effects on autologous progenitor cells. However, pharmacokinetic data suggest that the 24-hour waiting period may not be necessary, leading other institutions to allow stem cell infusion at shorter intervals. Objective: To analyze the data regarding TimeMel in patients with multiple myeloma undergoing ASCT, and to evaluate if that time is associated with platelet and neutrophil engraftment.

POPULATION:

Patients diagnosed with multiple myeloma who underwent ASCT, with melphalan-based conditioning, have been included. All patients were older than 18 years, with no upper age limit. Transplants performed between January 2023 and March 2025 were analyzed.

METHODS:

This is a retrospective observational study. Data were obtained through medical record analysis. The primary outcome was to evaluate the association between TimeMel and engraftment. Additional analyzed data included conditioning dose (melphalan 140 mg/m^2 vs. 200 mg/m^2), CD34 dose, occurrence of infections and mucositis. Associations between TimeMel and time to engraftment were assessed using the Kruskal–Wallis test. For subgroup comparisons (e.g., by CD34+ cell dose or melphalan dose), the Mann–Whitney U test was employed.

RESULTS:

A total of 99 patients were analyzed, stratified into three groups based on TimeMel: Group 1 (18–21h): 38 patients (38%); Group 2 (22–24h): 47 patients (48%); Group 3 (>24h): 14 patients (14%). About engraftment outcomes, the median time to neutrophil and platelet engraftment, across all groups, were 11 and 12 days, respectively, with no statistically significant difference ($p = 0.54$ and $p = 0.65$). No variance was found in the time to neutrophil engraftment between patients receiving different melphalan doses. Despite grade 3 mucositis being more frequent in group 3 (28%), no statistically

significant difference in mucositis severity across groups was seen (p=0.094). No association was found between TimeMel and hepatic or renal toxicity, or infection rates. In multivariate analysis, after adjusting for age, CD34 dose, and conditioning regimen, TimeMel remained non-significant for both neutrophil and platelet engraftment. 30-day mortality did not vary according to TimeMel; the median period of hospitalization was significatively higher in group 3. Faster engraftment was observed with higher CD34 doses (> 2.5 x 106/Kg; neutrophil: p=0.001; platelet: p=0.003).

CONCLUSION:

In this retrospective analysis, varying TimeMel did not significantly affect engraftment kinetics or transplant toxicity.

KEYWORDS: Autologous Stem Cell Transplant; Multiple Myeloma; Melphalan.

EXPERIENCES OF NURSES IN A BONE MARROW TRANSPLANT CENTER IN GOIÁS: EXPERIENCE REPORT

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INTRODUCTION:

Bone Marrow Transplantation (TMO) is a therapy used to treat oncohematological diseases, and is divided into autologous and allogeneic. Objective: To report the experiences of nurses regarding their professional performance in hematopoietic stem cell transplantation.

METHODOLOGY:

This is a report of professional experience, experienced by nurses who work in an autologous bone marrow transplant unit, developed in a reference hospital in Goiás, between April 2024 and March 2025.

RESULTS AND DISCUSSION:

During the observation period, 26 patients underwent transplantation. Regarding the oncohematological diagnoses, 73.1% were patients with Multiple Myeloma, 19.2% Non-Hodgkin Lymphoma and 7.7% Hodgkin Lymphoma. Of the patients who underwent collection, 64.5% reached the desired target of the first and only day of hematopoietic stem cell collection, and 34.6% did not obtain a satisfactory collection, requiring more than one day of collection. Among the main complications after hematopoietic stem cell transplantation, most patients presented fever, mucositis, intense asthenia, nausea, vomiting, dysphagia, odynophagia and diarrhea, resulting from the conditioning protocol. The success of the transplant is linked to the education and training of the team. It is necessary to standardize techniques to ensure the quality of the care provided.

CONCLUSION:

This experience provided not only knowledge, but also enhanced the development and improvement of technical skills for carrying out specific procedures in the nursing area, which enrich and contribute to training.

KEYWORDS: Nursing; Bone Marrow Transplantation; Hematopoietic Stem Cell Transplantation.

FRAILTY SCORE ANALYSIS OF MULTIPLE MYELOMA PATIENTS IN THE EVALUATION PRIOR TO AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Multiple Myeloma (MM) is the second most common hematological neoplasm and usually presents at older age. Use of high doses of melphalan followed by autologous hematopoietic stem cell transplantation (auto-HCT) remains the standard of care for treatment. In transplant eligibility evaluation, frailty assessment can predict increased probability of adverse reactions and worse outcomes. The International Myeloma Working Group (IMWG) developed a score to provide a reliable evaluation in the treatment decision-making process, which divides MM patients in fit, intermediate fit and unfit.

OBJECTIVES:

Assess the proportion of unfit MM patients according to IMWG score during the evaluation prior to auto-HCT, your relation with the age and with transplant ineligibility.

METHODS:

Retrospective with prospective arm, unicentric, observational, descriptive study based on review of medical records of MM patients evaluated for auto-HCT with use of IMWG score in an ambulatory of a tertiary hospital of northeast region of Brazil between 2023 and 2025. A total of 81 patients met the inclusion criteria.

RESULTS:

The sample had a female predominance with 53% of the cases evaluated, age at the time of the assessment varying from 31 to 73 years, a mean age of 59 years. 47 (58%) patients were considered fit, 26 (32%) patients, intermediate fit - 50% of them, younger than 60 years - and 8 (9%) - 62% of them, younger than 60 years - unfit, according to IMWG criteria. 14 (17%) individuals had the auto-HCT contraindicated, of those considered ineligible, almost the totality were from patients with frailty score intermediate, 8 (57%), or unfit, 4 (38%). In 10 (71%) patients, one of the described reasons to consider ineligibility to auto-HCT was the frailty score, always cited associated with one important comorbidity. Compared to other studies, our sample had a higher proportion of women and a lower mean age. The study that validated the IMWG frailty score had a higher percentage of unfit patients (30%), possibly because of higher mean age, 74 years.

CONCLUSION:

The frailty score of IMWG is an important tool to use during the evaluation prior auto-HCT for MM patients, considering the expressive prevalence of frailty syndrome in this population and helps to define the eligibility to this treatment strategy, expanding the discussion for beyond the age. Further analyses are important to validate this score in other and younger populations.

KEYWORDS:

Multiple Myeloma, frailty, autologous hematopoietic stem cell transplantation.

HEMATOPOIETIC STEM CELL INFUSION SAFETY CHECKLIST: STRATEGIES FOR ERROR REDUCTION AND IMPROVEMENT OF PATIENT SAFETY

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INTRODUCTION

Hematopoietic stem cell transplantation (HSCT) is a complex and high-risk procedure used in the treatment of various hematological, immunological, and oncological diseases. Given the complexity of the process, the occurrence of errors can seriously compromise patient safety. Therefore, it is essential to develop and implement systematic safety strategies. Among these strategies, the use of checklists stands out as an effective tool for process standardization and failure prevention. The application of a specific checklist for hematopoietic stem cell (HSC) infusion can significantly contribute to improving care quality and minimizing the risks associated with the procedure.

OBJECTIVE

To develop and implement a specific safety checklist for the HSC infusion process, aiming to reduce the occurrence of errors, standardize clinical practices, and increase the safety of patients undergoing transplantation.

METHODOLOGY

The study was conducted at a specialized HSCT institution accredited by the Joint Commission International, using a qualitative research design with case study elements. The methodology was divided into four main stages: literature review, risk analysis, checklist development, implementation, and evaluation.

RESULTS

The tool was structured into three phases: sign in, time out, and sign out. It included key elements such as: patient identification, verification of the Informed Consent Form, medical prescription, materials and equipment, hand hygiene and use of personal protective equipment, vital signs, administration of pre-infusion medications, allergies, functioning of the central venous access, team members, microbiological culture collection from the infusion bag, and adverse events involving the patient/equipment/materials. The checklist application showed significant positive impacts, such as a reduction in errors related to the pre-infusion steps, increased adherence to safety practices like double-checking and real-time recording of vital signs, enhanced safety perception among professionals involved, and improved communication between team members, especially during pre-infusion verification steps.

CONCLUSION

The implementation of a safety checklist for hematopoietic stem cell infusion proved to be an effective strategy for standardizing procedures, reducing errors, and increasing patient safety. Furthermore, it promoted an institutional safety culture and strengthened teamwork. The adoption of this tool should be encouraged as an essential practice in transplant centers, with periodic review and adaptation recommended to meet the specific needs of each service.

KEYWORDS: Patient Safety; Hematopoietic Stem Cell Transplantation; Clinical Checklist

IDENTIFICATION OF POOR MOBILIZERS AND THE USE OF PLERIXAFOR: EXPERIENCE FROM A NEWLY ESTABLISHED HEMATOPOIETIC STEM CELL TRANSPLANT CENTER

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INTRODUCTION:

Effective mobilization of hematopoietic stem cells (HSC) is crucial for the success of bone marrow transplantation. However, poor mobilizers can significantly impact the efficiency of HSC collection. Identifying poor mobilizers is essential for optimizing collection strategies and improving patient outcomes.

OBJECTIVE:

This study aims to evaluate the prevalence of poor mobilizers and assess the use of Plerixafor as a strategy to enhance HSC collection.

METHOD:

This retrospective single-center study analyzed data from 18 patients treated between September 2024 and May 2025. Patients were evaluated based on criteria established by the Italian Group for Bone Marrow Transplantation (GITMO) to identify poor mobilizers. All patients underwent mobilization with G-CSF at doses ranging from 10 to 14 µg/kg/day. Plerixafor was administered preemptively to patients with CD34+ counts <10 cells/µL on day 4 of mobilization. Two patients, classified as predicted poor mobilizers, did not undergo CD34+ count evaluations on day 4 due to logistical issues but still received Plerixafor.

RESULTS:

Twelve patients (66%) were categorized as predicted poor mobilizers based on GITMO criteria, which included factors such as previous therapy (Lenalidomide: n=4, Melfalano: n=1), age over 65 (n=3), and aggressive and refractory disease (n=5). Among the 9 patients who received Plerixafor, 2 did not have CD34+ counts on day 4 due to logistical challenges, 1 had a CD34+ count of 15 cells/µL but was considered potentially an early mobilizer, and 6 had CD34+ counts <10 cells/µL. Notably, only 1 patient in this group had not been classified as a predicted poor mobilizer. Of these 9 patients, only 1 was unable to mobilize and was confirmed as a proven poor mobilizer. Remarkably, the remaining 8 patients (88.9%) successfully achieved a collection exceeding 2 x 106 CD34+/kg cells, underscoring the effectiveness of Plerixafor in enhancing HSC mobilization in high-risk patients.

CONCLUSIONS:

Our experience suggests that early identification of poor mobilizers and the administration of Plerixafor can significantly improve HSC collection outcomes. Despite the small patient population, our results align with existing literature, indicating that adding Plerixafor to G-CSF increases the proportion of patients achieving CD34+ collection goals. Furthermore, in patients classified as poor mobilizers, Plerixafor can be used preemptively, demonstrating high success rates in obtaining adequate CD34+ cell numbers.

KEYWORDS: Hematopoietic Stem Cell Transplantation, Poor mobilizers, Plerixafor

IMPACT OF SARCOPENIA AND LOW MUSCLE ATTENUATION ON TOXICITY AND SURVIVAL AFTER AUTOLOGOUS STEM CELL TRANSPLANTATION IN ADULTS WITH LYMPHOMA

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BACKGROUND:

Autologous stem cell transplantation (ASCT) improves survival outcomes in lymphoma patients with chemotherapy-sensitive disease. Sarcopenia and low muscle attenuation (LMA) have been associated with adverse outcomes in oncology and ASCT settings; however, their prognostic significance in lymphoma patients undergoing ASCT warrants further investigation.

OBJECTIVE:

To determine if LMA and sarcopenia are predictive of ASCT toxicity, overall survival (OS), progression-free survival (PFS), and non-relapse mortality (NRM) in patients with lymphoma.

METHODS:

This retrospective cohort included patients who underwent ASCT between 2014 and 2017 at a single center. Pre-transplant computed tomography (CT) scans were used to assess skeletal muscle area at the third lumbar vertebra (L3). Sarcopenia was defined as a skeletal muscle index (SMI) <46 cm^2/m^2 for BMI <25 kg/m^2 or <51 cm^2/m^2 for BMI ≥25 kg/m^2 in men, and ≤41.5 cm^2/m^2 in women. LMA was classified as <41 Hounsfield units (HU) for BMI <25 kg/m^2 and <33 HU for BMI ≥25 kg/m^2. Toxicities were graded according to the Common Terminology Criteria for Adverse Events (CTCAE v4.0), and survival outcomes were analyzed using Kaplan-Meier and Cox regression models. Logistic regression evaluated the associations between body composition parameters and clinical outcomes.

RESULTS:

110 adults with lymphoma were included: 33% were sarcopenic and 29% had LMA, with 13% (14 patients) exhibiting both conditions (Table 1). Patients with LMA experienced significantly higher rates of grade 3-4 toxicities, including renal (multivariate Odds Ratio [OR]: 7.63; 95% CI 1.72–43.6; p=0.012), hepatic (OR: 7.88; 95% CI 1.28–9.67; p=0.003), and overall toxicity (OR: 3.43; 95% CI 1.28–9.67; p=0.016). At days +30 and +100 post-transplant, the cumulative incidences of NRM were 13% (95% CI: 3.9–26) and 19% (95% CI: 7.6–35) for patients with LMA, compared to 0% and 1.3% (95% CI: 0.1–6.2) in those without LMA (p<0.001), respectively (Fig. 1). In multivariate analysis, LMA was independently associated with higher NRM (Hazard Ratio [HR]: 9.51; 95% CI 1.11–81.7; p=0.04), although it was not significantly associated with PFS or OS. Sarcopenia was not significantly linked to peritransplant adverse events, OS, PFS, or NRM.

CONCLUSION:

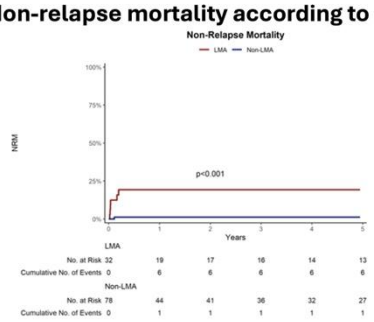
LMA, rather than sarcopenia, emerged as a strong predictor of post-transplant complications and increased NRM, suggesting that muscle quality is a more relevant biomarker than muscle mass alone in this setting. These findings support the integration of CT-based muscle attenuation analysis into pre-transplant risk assessment.

Table 1: Patient, disease and transplant characteristics

Characteristic	LMA vs. non-LMA			Sarcopenia vs. non-sarcopenia		
	Non-LMA, N = 78	LMA, N = 32	p-value	Non-Sarcopenic, N = 74	Sarcopenic, N = 36	p-value
Age, median (IQR)	39 (26; 50)	56 (46; 64)	**<0.001**	43 (28; 57)	47 (26; 56)	0.8
Sex			0.20			0.8
Male	54 (69%)	18 (56%)		49 (66%)	23 (64%)	
BMI – Category			0.60			**0.007**
Underweight	2 (2.6%)	0 (0%)		0 (0%)	2 (5.6%)	
Healthy Weight	28 (36%)	12 (38%)		24 (32%)	16 (44%)	
Overweight	31 (40%)	10 (31%)		26 (35%)	15 (42%)	
Obesity	17 (22%)	10 (31%)		24 (32%)	3 (8.3%)	
LDH (UI), median (IQR)	215 (162; 299)	212 (172; 352)	0.60	206 (159; 305)	225 (175; 347)	0.4
Unknown	4	2		3	3	
Albumin – Category			**0.020**			0.4
≥ 3.4	64 (86%)	20 (67%)		59 (83%)	25 (76%)	
< 3.4	10 (14%)	10 (33%)		12 (17%)	8 (24%)	
Unknown	4	2		3	3	
Disease			0.50			0.7
Hodgkin's lymphoma	32 (41%)	11 (34%)		28 (38%)	15 (42%)	
Non-Hodgkin's lymphoma	46 (59%)	21 (66%)		46 (62%)	21 (58%)	
HIV			0.20			**<0.001**
Positive	4 (5.3%)	4 (13%)		0 (0%)	8 (24%)	
Unknown	3	0		1	2	
Ann Arbor stage			0.60			0.2
I/II	16 (21%)	8 (25%)		19 (26%)	5 (14%)	
III/IV	62 (79%)	24 (75%)		55 (74%)	31 (86%)	
B symptoms at diagnosis	57 (73%)	22 (69%)	0.60	53 (72%)	26 (72%)	>0.9
Charlson comorbidity index			**<0.001**			**0.010**
0-2	52 (67%)	10 (31%)		48 (65%)	14 (39%)	
≥ 3	26 (33%)	22 (69%)		26 (35%)	22 (61%)	
Therapy line			0.30			0.2
1	21 (27%)	13 (41%)		26 (35%)	8 (22%)	
2	36 (46%)	13 (41%)		33 (45%)	16 (44%)	
≥ 3	21 (27%)	6 (19%)		15 (20%)	12 (33%)	

Characteristic	LMA vs. non-LMA			Sarcopenia vs. non-sarcopenia		
	Non-LMA, N = 78	LMA, N = 32	p-value	Non-Sarcopenic, N = 74	Sarcopenic, N = 36	p-value
Radiotherapy	19 (24%)	7 (23%)	0.80	18 (24%)	8 (23%)	0.9
Unknown	0	1		0	1	
Conditioning protocol			0.40			0.054
Carmustine/ Lomustine-based	64 (82%)	24 (75%)		63 (85%)	25 (69%)	
Busulfan-based	14 (18%)	8 (25%)		11 (15%)	11 (31%)	
Status of disease at ASCT			0.4			**0.010**
Complete response	45 (59%)	21 (68%)		52 (71%)	14 (41%)	
Partial response	27 (36%)	7 (23%)		17 (23%)	17 (50%)	
Refractory	4 (5.3%)	3 (9.7%)		4 (5.5%)	3 (8.8%)	
Unknown	2	1		1	2	
CD34 cells infused ($x10^6$/Kg), median(IQR)	4.8 (3.6; 9.0)	4.3 (3.3; 5.8)	0.13	4.5 (3.4; 9.3)	4.9 (3.9; 7.2)	>0.9

Figure 1: Non-relapse mortality according to LMA status



INITIAL RESULTS OF MOBILIZATION WITH FILGRASTIM AS A SINGLE AGENT IN AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION AT A PUBLIC HOSPITAL IN BRAZIL

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INTRODUCTION:

The first autologous hematopoietic stem cell transplant (ASCT) was performed in our center less than 2 years ago. Peripheral hematopoietic stem cells are the main source for ASCT. Previous data analyzing traditional mobilization protocols using single agent G-CSF reported failure rates up to 38%. The plerixafor + G-CSF has been associated with improved cell mobilization and lower failure when compared to chemotherapy + G-CSF or G-CSF alone but is not available in public health system (SUS) in Brazil.

OBJECTIVES:

Primary objective was to determine the mobilization rate for G-CSF alone in ASCT at our center. Infection rates and time to engraftment were secondary endpoints.

CASUISTIC:

23 patients underwent mobilization with G-CSF. Median age was 58 years, 52.2% were male, 78.3% had plasma cell disease (17 multiple myeloma and 1 solitary plasmacytoma) and 12.7% were lymphomas, 2 diffuse large B-cell lymphoma (DLBCL), 2 Hodgkin lymphoma (HL) and 1 Waldenström's macroglobulinemia.

METHODS:

Patients were consecutively included from December 2023 to May 2025. Data were retrospectively collected and analyzed from patients electronic medical records and electronic laboratory system. G-CSF 10µg/kg for five days was used in the first mobilization attempt and in failure cases, vinorelbine + G-CSF was performed. $CD34^+$ cell collection of $<2\times10^6$ $CD34^+$/kg was considered mobilization failure. One patient was excluded because collection was aborted due to COVID-19.

RESULTS:

Mobilization rate with GCSF alone was 95.7%. The median peripheral blood $CD34^+$ count prior to collection was 22.3 cells/mm^3 (1.2-129/mm^3). The median $CD34^+$ count was 2.095×10^6 $CD34^+$/kg ($1.03\text{-}8.5\times10^6$ $CD34^+$/kg) in the first collection and 1.01×10^6 $CD34^+$/kg ($0.57\text{-}1.95\times10^6$ $CD34^+$/kg) in the second collection. Eight patients (34.8%) required two collections. One failed and was subsequently mobilized with vinorelbine-based protocol. Overall, the median $CD34^+$ count per patient was 2.765×10^6 $CD34^+$/kg ($1.53\text{-}8.5\times10^6$) with G-CSF alone. The median time to neutrophil and platelet engraftment was 11 days (range 9 to 13) and 11 days (range

9 to 14), respectively. The median hospital stay was 16 days (range 14 to 30). 90.5% (19/21) of the patients who mobilized with G-CSF and underwent transplantation received therapeutic antibiotics due to fever and 28.57% (6/21) had positive blood cultures, mostly with gram-positive bacteria commonly found on the skin which could be related to central vein catheter or to contamination. There were no severe infections, no need for ICU admission, and no transplant-related deaths.

CONCLUSIONS:

In this study, ASCT with hematopoietic stem cell mobilization using filgrastim (G-CSF) alone showed high collection success rates (95.7%), with a median engraftment time of 11 days and no severe infectious complications, supporting its efficacy and safety for use in the public healthcare system (SUS) in Brazil.

KEYWORDS: Mobilization, G-CSF and autologous hematopoietic stem cell transplant.

LYMPHOMA HODGKIN AND AUTOLOGOUS STEM CELL TRANSPLANT - A REPORT OF 20 YEAR EXPERIENCE AT A SINGLE CENTER -

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Myeloablative chemotherapy with Autologous Stem Cell Rescue (ASCR) is an approach for patients with Hodgkin Lymphoma (HL) who develop refractory disease during therapy or relapsed disease within 1 year after completing therapy; recurrent, extensive disease after one year of completing therapy or for those with recurrent disease after initial therapy that included intensive multiagent chemotherapy and radiation therapy (RT) . In addition to ASCR, high risk relapsed HL may benefit from brentuximab vedotin (BV) as a consolidation treatment after transplant.

OBJECTIVE

To describe experience with HL R/R treatment with ASCR at a pediatric oncology/hematology center over the last 20 years.

METHODS

Retrospective analysis of HL patients who underwent ASCR between 2005 and 2025. All patients received first-line, rescue, RT or maintenance treatment after ASCT at same hospital. ASCR were performed at different hospitals. No uniform treatment was used for first line or rescue chemotherapy. After 2020, patients with high-risk relapses (refractory disease, relapse within one year after therapy, bulky disease, or recurrence at sites of previous radiotherapy) received one year of brentuximab after ASCT. Almost all treated in the Public Health System.

RESULTS:

From 2005 to 2025, 331 children and adolescents were diagnosed with HL. Of those, 31 (10%) underwent ASCT. Approximately 22% were 10 years old or older. Most had high-risk disease (67%). Eight (22%) had relapsed or were refractory to first-line therapy. Nearly 70% received RT before ASCT. Patients evaluated before transplant without PET-CT didn´t have worse EFS or OS. The most used conditioning regimen was BEAM (58%), and there were no significant differences in EFS/OS between conditioning types (Fig1). Consolidation therapy with RT was performed in nine children (29%) and with BV in ten patients (32%). Only 1 child developed a second malignancy (AML). Six patients relapsed after ASCT with multiple treatment strategies (allo SCT, RT and BV), four are alive. Non-relapse mortality (NRM) was 16%, primarily due to sepsis. Four more children (12%) died from the disease. Three patients who underwent ASCT with active disease did not survive (one due to disease progression and the others due to infection). The EFS was 53.1%, and the OS was 67.4% . (Table 1 and Fig2).

DISCUSSION:

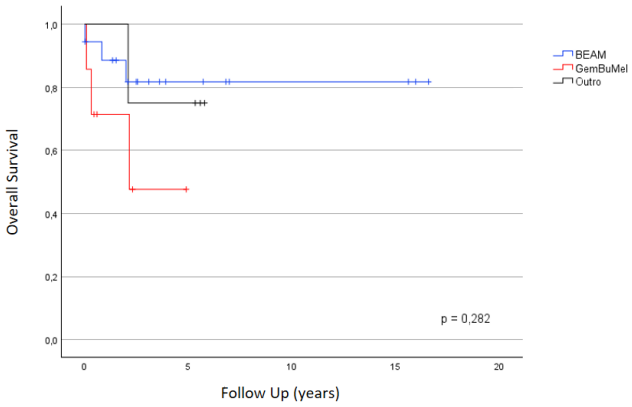
Over the years HL treatment has developed to reduce toxicity by classifying patients into risk groups, which has led to a reduction in the intensity of radiotherapy (RT) and the use of alkylating agentes. Nevertheless, infections remain the primary cause of NRM in patients requiring ASCT as consolidation therapy in our coort. Other therapies can contribute to reduce toxicity by decreasing RT before and after ASCT and to rescue those who relapsed after ASCT. Using target therapy and/or checkpoint inhibitors may lead to less toxicity, improved EFS, and consequently, better OS. The challenge is to incorporate it at Public Health System.

TABLE1 – Demografic data

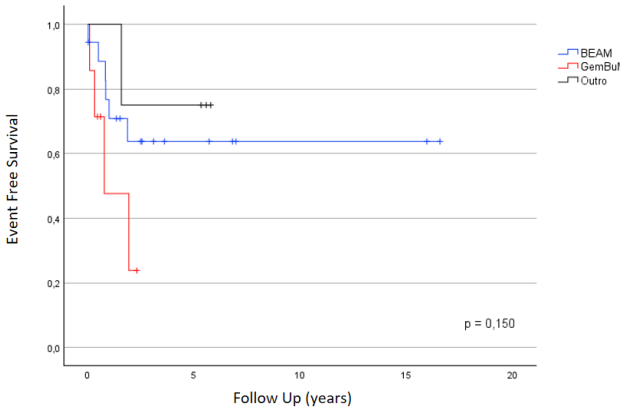
Demography Data	N	%
Total	31	
Gender		
Male	18	58,06
Female	13	41,94
Age (years)		
Median = 16		
< 05	1	3,23
>= 5 a < 10	4	12,90
>=10 a < 15	7	22,58
Stage		
I	2	6,45
II	6	19,35
III	8	25,81
IV	15	48,39
Risk Group at 1st treatment		
Low	3	9,68
Intermediate	7	22,58
High	21	67,74
Cellular Classification		
Nodular Sclerosing	27	87,10
Nodular lymphocite-predominant	3	9,68
No Information	1	3,23
Progression during 1st treatment		
Yes	7	22,58
No	24	72,42
Remission pré HSCT		
Yes	28	90,32
No	3	9,68
PET CT before HSCT		
Yes	20	64,52
No	8	25,81
No information	3	9,68
Radiotherapy before HSCT		
Yes	22	70,97
No	9	29,03
Conditioning		
BEAM	18	58,06
GemBuMel	7	22,58
Another	4	12,90
No Information	2	6,45

Radiotherapy post HSCT		
Yes	9	29,03
No	22	70,97
Brentuximab post HSCT		
Yes	10	32,26
No	21	67,74
Relapse after HSCT		
Yes	6	19,35
No	25	80,65
Second Malignancy		
Yes	1	3,23
No	30	96,77
Cause of Death		
Non relapse Mortality	5	16,13
Relapse	4	12,90
Alive	22	70,97
Follow Up		
3,3 years (median)		

FIG1 OS and EFS according to conditioning chemotherapy

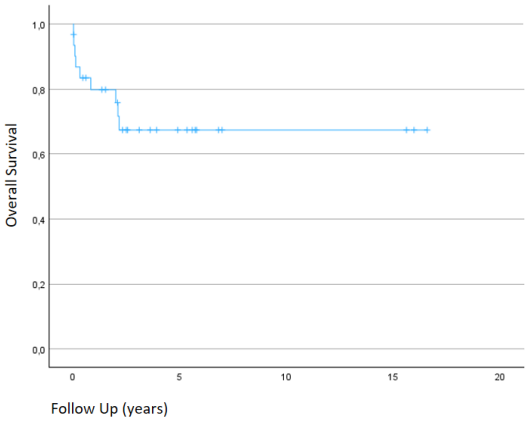


BEAM OS = 100% (N = 18 Alive = 15)
GemBuMel OS = 100% (N = 7 Alive = 4)
Another OS = 66,7% ± 27,2% (N = 4 Alive = 3)

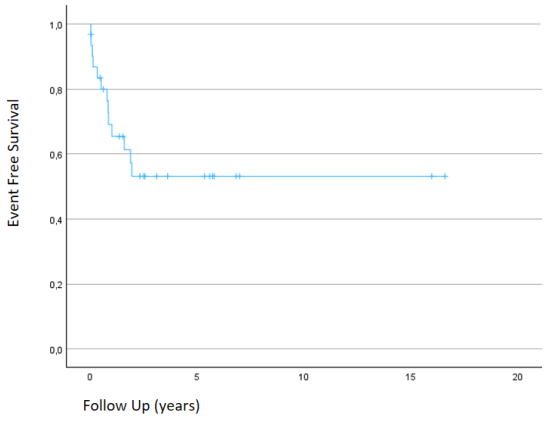


BEAM EFS = 63,8% ± 12,0% (N = 18 Remission = 12)
GemBuMel EFS = 23,8% ± 20,3% (N = 7 Remission = 3)
Outro EFS = 75,0% ± 21,7% (N = 4 Remission = 3)

FIG2 OS and EFS



OS (5y) = 67,4% ± 9,1% (N = 31 Alive = 22)



EFS (5y) = 53,1% ± 9,6% (N = 31 Complete Remission= 18)

MOBILIZATION PROFILE OF PERIPHERAL CD34+ CELLS IN PATIENTS WITH MULTIPLE MYELOMA UNDERGOING FRESH AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Patients with Multiple Myeloma are usually candidates for autologous hematopoietic stem cell transplantation (HSCT), except when they meet ineligibility criteria such as advanced age (>75 years), significant comorbidities or disease refractory to primary treatment. To allow for hematopoietic stem cell (HSC) collection by apheresis, patients undergo mobilization on an outpatient basis with filgrastim for five to seven days, but the response to mobilization is different among patients, and some require additional drugs for mobilization, such as plerixafor.

OBJECTIVE:

To evaluate the variation in the number of HSC (CD34+ cells) on the day before collection compared to the day of collection in patients who received filgrastim or filgrastim+plerixafor.

METHOD:

Retrospective study evaluating the number of CD34+ cells in patients with Multiple Myeloma undergoing autologous HSCT at our institution from December 2021 until April 2025. The CD34+ cell count was performed by flow cytometry by the hospital's Central Laboratory using FACSCanto II cytometer.

RESULTS:

In this period, 64 fresh autologous HSCT were performed, and 57 patients (89.1%) reached the target of 2x106 CD34+ cells/kg in just one day of collection, while 7 patients required a second day of collection. A total of 71 HSC collections were performed using the Spectra Optia apheresis equipment. Of these 64 patients, 7 (10.9%) did not respond adequately to mobilization with filgrastim alone considering the number of peripheral CD34+ cells on the day before collection and, therefore, met the criteria for the use of plerixafor. Also, one patient received plerixafor only for his second day of collection, so that the target of 2x106 CD34+ cells/kg could be reached. When evaluating the increase in peripheral CD34+ cells/ul between the day before and the day of collection, it was possible to see an average increase of 167% (SD=51%; 71% to 386%; n=55) in patients who only used filgrastim, while the average increase in patients who used filgrastim+plerixafor was 457% (SD=220%; 270% to 888%; n=8). Two patients were excluded due to lack of CD34+ cell count data on the day of collection.

CONCLUSIONS:

Most patients with Multiple Myeloma were able to complete the collection of at least 2x106 CD34+ cells/kg in just one day and only using filgrastim. The patients with peripheral CD34+ count close to 10 cells/ul on the day before collection had almost double the number of CD34+ cells/ul on the following day even when using filgrastim alone. However, the use of plerixafor has a more noticeable impact on the mobilization of HSC to the peripheral blood, since, on average, it more than quadrupled the number of CD34+ cells/ul compared to the previous day, which corroborates with the findings described in the literature.

KEYWORDS: Hematopoietic Stem Cell Transplant, Multiple Myeloma, Stem Cell Mobilization.

NAÏVE REGULATORY T-CELLS AND IL-10 LEVELS CORRELATE WITH FAVORABLE CLINICAL OUTCOMES IN TYPE 1 DIABETES PATIENTS TREATED WITH AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Most Type 1 Diabetes (T1D) patients treated with autologous Hematopoietic Stem Cell Transplantation (AHSCT) achieve insulin independence, remaining free of exogenous insulin replacement for a median time of 3 years. Mechanistic studies have suggested that clinical outcomes are associated with improved self-tolerance and immunoregulation.

OBJECTIVE:

To assess IL-10 serum levels and frequencies of regulatory T cells (Tregs) in the peripheral blood, before and after AHSCT, in order to better understand patient clinical outcomes.

METHODS:

Blood samples collected from 9 T1D patients had their Peripheral Blood Mononuclear Cells (PBMCs) assessed by Flow Cytometry to identify Treg populations (CD3+CD4+CD25+FOXP3+) and naive Treg cells (CD3+CD4+CD25+FOXP3+CD45RA+). Plasmatic cytokine dosage was performed using ELISA kits. Results were retrospectively correlated with the patients' clinical outcomes of short (under 3 years) and long insulin independence (over 3 years).

RESULTS:

Most patients were male (66.6%) with a median age of 18.6 years (range 16–23). Following AHSCT, patients became insulin-free and showed increased C-peptide levels. At 360 days post-transplant, serum IL-10 concentrations were significantly higher in patients with longer insulin independence compared to those with short duration of insulin independence ($p < 0.05$; Figure 1). IL-10 serum concentrations correlated positively with C-peptide concentrations ($R^2 = 0.66$), suggesting a relationship with preserved endogenous insulin production. Interestingly, IL-10 concentrations negatively correlated with the overall frequency of regulatory T cells (Tregs) in the PBMCs. However, the frequency of naïve Tregs increased significantly at day 100 post-transplant ($p < 0.05$; Figure 2) and showed a positive correlation with IL-10 ($R^2 = 0.70$) and C-peptide levels ($R^2 = 0.43$) (Figure 3).

CONCLUSIONS:

Unexpectedly, our results indicate that the concentrations of IL-10, an anti-inflammatory cytokine, inversely correlate with the frequencies of Treg cells. However, we also show that within the Treg population there is a naïve Treg subpopulation that strongly and positively correlates with IL-10 serum concentrations. We believe that IL-10 could be produced by naïve (CD45RA+) Treg cells, which are shown to increase after AHSCT. Importantly, naïve Treg frequencies and IL-10 levels are associated with favorable clinical outcomes of C-Peptide levels that reflect endogenous insulin production. Additionally, when patients were clustered according to duration of insulin independence, the long-term response patients tended to present higher concentrations of IL-10 in serum samples, at all time points, than the short-term response patients. These results show that the naïve Treg population and IL-10 concentrations are associated with favorable clinical outcomes of T1D treated with AHSCT.

KEYWORDS: Type 1 Diabetes, Autologous Stem Cell Transplantation, Immunoregulation

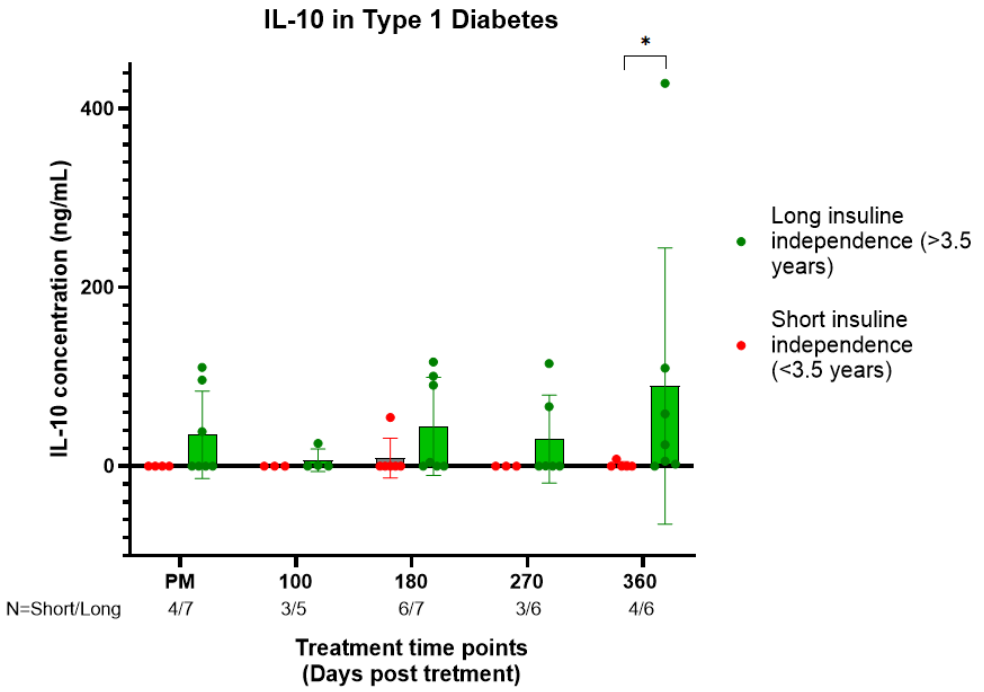

Figure 1: IL-10 concentration (ng/mL) in serum samples of T1D patients in different treament time points: PM (pre-mobilization), 100, 180, 270 and 360 days post transplant. Patients are separated in response groups: long term insulin independence (green) and short term insulin independence (red).

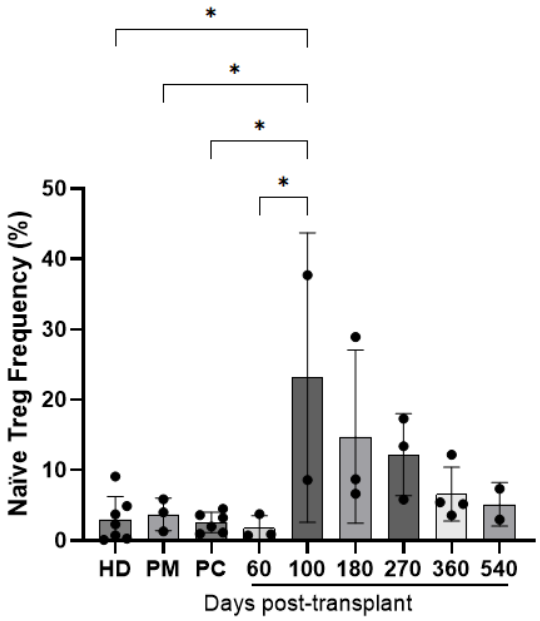

Figure 2: Naive Regulatory T Cell (CD3+CD4+CD25+FOXP3+CD45RA+) frequency in T1D patients in differente time points. HD (Healthy Donor); PM (pre-mobilization); PC (pre-conditioning).

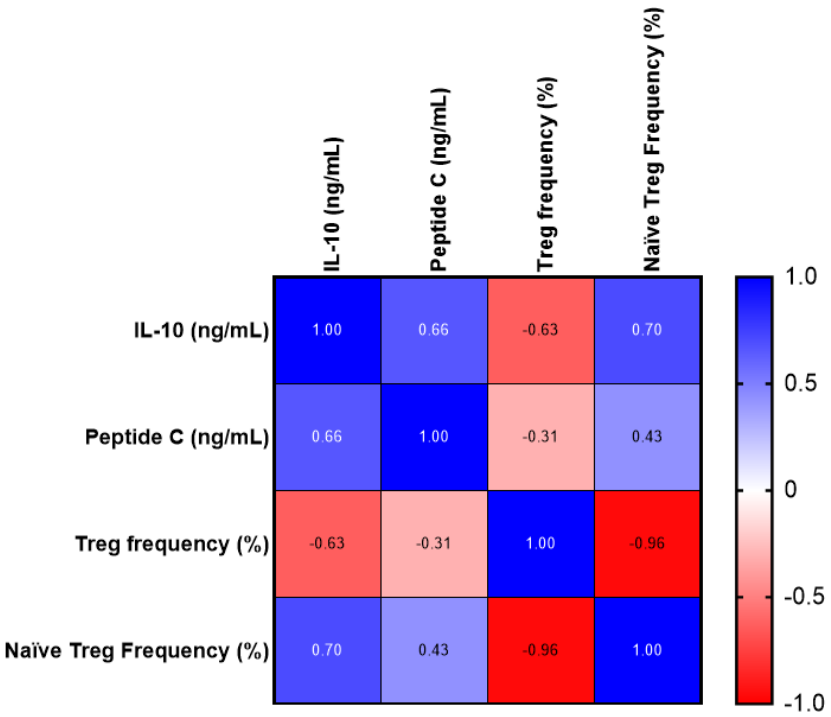

Figure 3: Heatmap representation of correlation analysis of IL-10 concentration (ng/mL), Peptide C concentration (ng/mL), Regulatory T Cell (Treg) frequency (%) and naïve Treg frequency (%).

Keywords: Type 1 Diabetes, Autologous Stem Cell Transplantation, Immunoregulation

QUALITY OF LIFE IN MULTIPLE MYELOMA PATIENTS UNDERGOING AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION: A LONGITUDINAL PROSPECTIVE STUDY

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INTRODUCTION:

Multiple myeloma (MM) is the second most common hematologic malignancy, accounting for approximately 1.8% of all new cancer cases worldwide. It significantly compromises patients' quality of life (QoL) due to symptoms such as anemia, renal dysfunction, hypercalcemia, osteolytic bone lesions, and pathological fractures. Standard treatment typically includes induction chemotherapy followed by autologous hematopoietic stem cell transplantation (AHSCT) for eligible patients. AHSCT consists of high-dose chemotherapy followed by the reinfusion of the patient's own hematopoietic stem cells, and it is typically divided into three phases: pre-transplant, transplant, and post-transplant. Despite the clinical importance of QoL assessment in MM, data from the Brazilian context remain scarce, revealing a significant knowledge gap. This study seeks to address this gap and provide foundational data that may support future cost-utility analyses based on QoL outcomes in Brazil.

OBJECTIVE:

To estimate the quality of life of multiple myeloma patients undergoing treatment at a hospital in São Paulo, Brazil.

METHODS:

This is a longitudinal, prospective cohort study with a quantitative approach, conducted in the bone marrow transplant (BMT) unit. The study will include MM patients undergoing AHSCT over a 1.5-year period from the beginning of data collection. A convenience sampling method will be used to recruit approximately 150 patients from the pre-transplant outpatient clinic.Data collection will involve the administration of sociodemographic questionnaires and validated QoL instruments, including the EQ-5D, EORTC QLQ-C30, and EORTC QLQ-MY20, at three time points: pre-transplant, intra-transplant, and post-transplant. Data will be organized in Excel spreadsheets and analyzed using SPSS version 26, following the scoring and interpretation guidelines of the respective instruments to identify correlations between patient QoL and study variables.

RESULTS:

The study aims to estimate the quality of life of MM patients undergoing AHSCT, providing valuable insights into patient experiences across the transplant process and generating data that could support future economic evaluations.

CONCLUSIONS:

By addressing a critical gap in the Brazilian literature, this study may contribute to improved clinical decision-making and the development of cost-utility models based on local QoL data.

KEYWORDS:

Multiple Myeloma; Quality of Life; Autologous Hematopoietic Stem Cell Transplantation

REAL-WORLD DATA ON AUTOLOGOUS TRANSPLANT IN PATIENTS WITH PLASMA CELL NEOPLASMS

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INTRODUCTION:

Autologous hematopoietic stem cell transplantation (auto-HSCT) is an established therapeutic approach for plasma cell neoplasms, including multiple myeloma and light-chain amyloidosis. However, post-transplant survival and factors associated with clinical outcomes require ongoing investigation, particularly in real-world settings.

OBJECTIVE:

To evaluate post-transplant survival and demographic characteristics of patients with plasma cell neoplasms who underwent auto-HSCT at a single center.

METHODS:

An observational, single-center study including patients diagnosed with multiple myeloma or light-chain amyloidosis who underwent auto-HSCT between April 2022 and September 2024. Overall survival was analyzed using the Kaplan-Meier method, measuring the time from transplantation to death or last follow-up.

RESULTS:

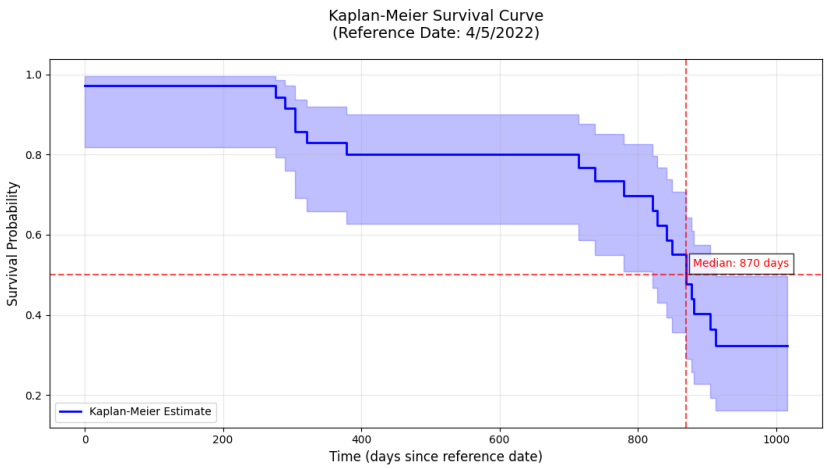
Eighteen patients were evaluated, with a median age of 57 years. Most were female (83.3%, n=15), and diagnoses included multiple myeloma (94.4%, n=17) and amyloidosis (5.6%, n=1). Eleven percent of patients (n=2) had undergone prior auto-HSCT, and 11% (n=2) had comorbidities (diabetes). The median follow-up was 186 days, with two early deaths (34 and 62 days post-transplant) attributed to infectious complications. Survival analysis demonstrated an overall survival rate of 88.9% during the study period.

CONCLUSION:

The results highlight the importance of strategies to reduce early post-transplant infectious complications and improve clinical outcomes. The study reinforces the need for rigorous monitoring and personalized interventions in this population.

KEYWORDS:

Multiple myeloma, Amyloidosis, Hematopoietic stem cell transplantation



SEVERE GASTROINTESTINAL BLEEDING IN A PATIENT WITH AL AMYLOIDOSIS UNDERGOING AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION: A CASE REPORT AND LITERATURE REVIEW

Mathias Regis Modesto[1], Carla Campos Miranda[1], Ricardo Romano[1], Guilherme Cecchetti[1], Silvio Henrique Vieira Freitas[1], Marcos Roberto Curcio Pereira[1], Gabriela Ugioni Duarte[1], Guilherme Lourenço de Macedo[1], Ediely Layana Oliveira Coletto[1], Talita Carvalho Siqueira[1], Nyara Walleska Maciel de França[1], Ana Paula de Azambuja[1], Rafael Marchesini[1], Felipe Borges Patrício[1], Daniela Setubal[1], Samir Nabhan[1], Glaucia Tagliari[1], Miguel Pedro de Queiroz Neto[1], Caroline Bonamin Sola[1],Vaneuza Araújo Moreira Funke[1]

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INTRODUCTION:

Systemic light-chain amyloidosis (AL) is a multisystem disorder characterized by the extracellular deposition of insoluble amyloid fibrils, leading to progressive organ dysfunction. Autologous hematopoietic stem cell transplantation (AHSCT), following high dose melphalan conditioning, remains the standard treatment for eligible patients. However, the degree of organ involvement significantly increases the risk of complications, including bleeding, renal injury, infection, and early mortality.

OBJECTIVE:

To report a case of severe gastrointestinal bleeding in a patient with AL amyloidosis who underwent AHSCT, discussing the underlying pathophysiological mechanisms and clinical management challenges, considering the current literature.

METHODS:

This is a descriptive study based on a retrospective review of the medical record of a patient diagnosed with AL amyloidosis (Mayo 2004 IIIA and European 2015 IIIA), who underwent AHSCT in March 2024. A systematic literature review was conducted using PUBMED, MEDLINE, and Science Direct databases, focusing on hemorrhagic complications associated with AHSCT in amyloidosis patients.

RESULTS:

A 41-year-old female patient with multiorgan involvement due to AL amyloidosis. Cardiac: initial ejection fraction of 28%, improved to 69.8% after pre-AHSCT treatment; renal: significant proteinuria (1,477 mg/dL) and elevated beta-2-microglobulin (9.57 mg/L); and hepatic: persistently elevated alkaline phosphatase. After receiving melphalan 200 mg/m^2 as conditioning, the patient developed massive enterorrhagia and grade 4 gastrointestinal mucositis during the aplasia phase. She required intensive transfusion support and ICU care, with refractory hemodynamic instability and the need for multiple vasopressors. Physical examination revealed hepatomegaly and oral mucosal ulcers. Progressive renal and hepatic function deterioration was noted. Despite supportive measures, the patient progressed to septic shock and died. The gastrointestinal bleeding was considered multifactorial, resulting from: Amyloid infiltration of vascular and submucosal layers, leading

to loss of vascular integrity; direct mucosal damage caused by high-dose melphalan chemotherapy; Thrombocytopenia associated with marrow aplasia and temporary discontinuation of filgrastim; hemostatic dysfunction due to renal and hepatic impairment, contributing to coagulopathy and platelet dysfunction; and splanchic hypoperfusion in the context of vasodilatory shock and mechanical ventilation.

CONCLUSION:

This case highlights the complexity of managing AL amyloidosis patients undergoing AHSCT, particularly regarding bleeding complications. Gastrointestinal bleeding in this context results from a combination of anatomical, therapeutic, and hemodynamic factors. Rigorous assessment of transplant eligibility, continuous monitoring, and intensive multidisciplinary management are essential to mitigate the high morbidity and mortality associated with these patients.

KEYWORDS:

Amyloidosis; Autologous Stem Cell Transplantation; Gastrointestinal Hemorrhage

THERAPEUTIC WINDOW OF BONE MARROW TRANSPLANTATION IN MANTLE CELL LYMPHOMA: CHALLENGES IN OPTIMIZATION

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INTRODUCTION:

Mantle cell lymphoma (MCL) is an aggressive B-cell neoplasm with a heterogeneous clinical course, low cure rates, and median survival of 3–5 years without intensive treatment. The introduction of high-dose cytarabine-based chemotherapy, followed by autologous hematopoietic stem cell transplantation (ASCT) in first complete remission and maintenance with rituximab, significantly improved progression-free survival (PFS) and overall survival (OS). However, defining the ideal therapeutic window for ASCT—the optimal period between remission and transplant—remains a key challenge. Disease heterogeneity, variable responses to induction, and the emergence of targeted therapies demand a strategic approach to balance relapse risk and transplant-related toxicity. Optimizing this window is critical to maximize benefits and minimize failure due to delays or suboptimal timing. Objectives: This study analyzes the evidence on the ideal therapeutic window for autologous transplantation in mantle cell lymphoma, considering its impact on clinical outcomes and challenges in defining the most appropriate time for its performance.

METHODS:

Literature review in PubMed, ASH Publications, and SciELO using the terms: "mantle cell lymphoma," "autologous stem cell transplantation," "therapeutic window," and "first remission." Included English-language articles (2020–2025) on the impact of ASCT timing in MCL.

RESULTS:

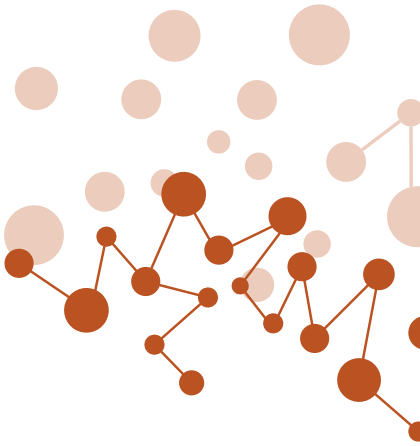
The introduction of chemotherapy with cytarabine and ASCT in the first remission of MCL increased progression-free survival and overall survival. Maintenance with rituximab post-transplantation further extended the duration of remissions. Despite the advancement of targeted therapies, the ideal timing for ASCT remains crucial, as delays may compromise the prognosis due to increased tumor resistance. The clinical heterogeneity of MCL demands an individualized approach, balancing the risks of transplantation and the efficacy of emerging therapies. The ideal therapeutic window remains essential, requiring a balance between the risk of progression and the toxicity of the procedure, considering the biological heterogeneity of MCL.

CONCLUSION:

Autologous hematopoietic stem cell transplantation (ASCT) in first remission, associated with intensified chemotherapy with cytarabine and maintenance with rituximab, is the strategy that most prolongs progression-free and overall survival in mantle cell lymphoma (MCL) to date. The optimal therapeutic window is critical but challenging due to the heterogeneity of the disease and the emergence of targeted therapies. Despite advances, MCL remains incurable in most cases, and optimizing the timing of transplantation, integrated with emerging therapies, is essential to improve outcomes. The approach must be individualized, and new studies are essential to improve this integration.

KEYWORDS: Mantle Cell Lymphoma; Autologous Transplant; Therapeutic Window

PEDIATRIC HCT



ACHIEVING SUCCESS THROUGH COOPERATION: STRATAGY TO PERFORM AN HAPLOIDENTICAL STEM CELL TRANSPLANTAT (HCT) FOR SICKLE CELL DISEASE IN A PATIENT WITH SEVERE HEMOPHILIA A

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INTRODUCTION:

Hemophilia A is an inherited, X-linked bleeding disorder characterized by factor VIII deficiency, with an incidence of approximately 1 in 5,000 men. Sickle cell disease (SCD) is a genetic disease of hemoglobin with an autosomal recessive pattern, one of the most prevalent in the world, especially in populations of African origin, with high morbidity and mortality. The coexistence of severe hemophilia A (<1% factor VIII activity) and sickle cell anemia (Hb SS) is extremely rare. To date, to the best of our knowledge, there are no descriptions of HCT in patients with concomitant hemophilia A and SCD.

OUR OBJECTIVE

is to describe the strategy to perform a successful HCT in a hemophilic patient. Casuistic: One patient with both hemophilia A and SCD.

METHOD:

As specific hemophilia procedures, factor VIII was replaced during transplantation 1,000 U 12/12h until platelet engraftment and daily until D+50, with an additional dose in days of invasive procedures. Transfusion support to maintain platelets always > 100,000/mm^3

RESULT:

A 21-year-old male patient was diagnosed with severe hemophilia A and sickle cell anemia since 3 months of age. Prophylactic factor VIII (3x/week) and hydroxyurea (since 11 years). Relevant past medical history: hemarthroses, femoral head osteonecrosis, multiple vaso-occlusive crises, acute chest syndrome, chronic obstructive pulmonary disease, resolved dialytic acute renal failure, moderate hepatic iron deposition, lung abscesses, hepatic sequestration, splenic sequestration followed by splenectomy, cholelithiasis followed by cholecystectomy, pyelonephritis, corpus callosum microhemorrhage, and hypogonadism. Without an HLA-identical donor, haploidentical HCT was performed with the mother (AS genotype) with conventional ATG, thiotepa, fludarabine, cyclophosphamide and

mesna, TBI 200 cGy and GVHD prophylaxis with post-transplant cyclophosphamide D+3 and D+4, followed by sirolimus and mycophenolate mofetil. He had neutrophil engraftment on D+21 and platelet engraftment on D+57 and has always had complete donor chimerism. Post-HCT complications were grade II acute GVHD of the skin and gut, chronic ocular, oral, cutaneous, and hepatic GVHD treated with corticosteroid and ruxolitinib for 15 months with complete response. He also had HHV-6 reactivation with probable encephalitis, gut HHV-7, COVID-19, Coxsackie B infection, renal lithiasis. Now, 3 years post HCT the patient maintains hemoglobin of 15 g/dL, does not have any pain crises, works and maintains prophylactic use of factor VIII 3x/week and is still on sirolimus. In conclusion, this is the first report of HCT SCD in a patient with coexistence of severe hemophilia A. Follow-up together with hematology and other specialties was essential for the safe conduct of the transplant and favorable evolution. Individualized management, with attention to hemostatic support, allowed the transplant to be performed safely in this rare context.

KEYWORDS: Sickle cell disease; hemophilia A; haploidentical

ACUTE AND CHRONIC GRAFT-VERSUS-HOST DISEASE IN PEDIATRICS: RISK FACTORS AND MANAGEMENT STRATEGIES

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INTRODUCTION:

Allogeneic hematopoietic stem cell transplantation (HSCT) remains a curative option for numerous malignant and non-malignant pediatric disorders. Despite advances in supportive care and transplant techniques, graft-versus-host disease (GVHD) continues to be a leading cause of non-relapse morbidity and mortality in this population. Acute GVHD (aGVHD) typically affects the skin, liver, and gastrointestinal tract, whereas chronic GVHD (cGVHD) can present with multisystemic involvement and autoimmune-like features. Pediatric patients exhibit distinct immunological responses and clinical presentations compared to adults, underscoring the need for tailored risk assessment and management strategies. Objective: To synthesize current evidence on risk factors, clinical characteristics, diagnostic criteria, treatment approaches, and preventive strategies for aGVHD and cGVHD in pediatric HSCT recipients, with an emphasis on emerging therapies and outcomes.

METHODOLOGY:

A narrative literature review was conducted on PubMed, Scopus, and Web of Science, covering studies published from January 2019 to April 2024, using the descriptors "pediatric GVHD," "acute GVHD" and "chronic GVHD".

RESULTS:

Significant risk factors for GVHD in children include HLA mismatch, unrelated or female donor to male recipient, older donor age, peripheral blood stem cell source, and myeloablative conditioning. Corticosteroids remain the mainstay of first-line therapy for both aGVHD and cGVHD. In steroid-refractory cases, ruxolitinib has demonstrated encouraging response rates and a manageable safety profile. Fecal microbiota transplantation (FMT) has emerged as a promising intervention for gastrointestinal GVHD, with documented clinical improvement in pediatric cohorts. Furthermore, cell-based therapies, including regulatory T cell (Treg) infusions and low-dose interleukin-2, are under investigation for their capacity to restore immune tolerance. Preventive approaches such as post-transplant cyclophosphamide, T-cell depletion, and abatacept in HLA-mismatched settings are gaining traction in clinical practice. GVHD severity correlates strongly with reduced quality of life and long-term functional impairment, reinforcing the importance of early intervention and personalized management.

CONCLUSION:

GVHD remains a critical challenge in pediatric HSCT, with complex pathophysiology and variable clinical trajectories. The incorporation of targeted therapies and immune-modulatory strategies into individualized care protocols has improved outcomes, yet substantial gaps persist in early detection, long-term safety, and therapeutic accessibility. Future progress depends on continued pediatric-focused research, development of evidence-based guidelines, and broader access to novel therapies capable of reducing GVHD-related morbidity and improving long-term survivorship.

KEYWORDS: Graft-versus-host disease, pediatric disorders, cell-based therapies

ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTS (HCT) CONTINUES TO HAVE 40% OVERALL SURVIVAL IN CHILDREN WITH REFRACTORY ACUTE MYELOGENOUS LEUKEMIA (AML), WHO SHOULD NOT BE DENIED THERAPY

Ana Carolina Ribeiro Correa[1], Luciana dos Santos Domingues[1], Maria Gabriela Matos Alves Dias[1], Laís Lima Quintino[1], Roseane Vasconcelos Gouveia[1,2], Valeria Cortez Ginani[1,2], Raisa Machado Amaral[1], Erica Almeida Viana[1], Ana Claudia Ramos Donatelli[1], Aline Ferrari Martins[1], Nathalia Gonçalves Rissardi[1], Yohanna Bastani de Mattos[1], Maria Estela Shiroma[1], Lahis Cardoso de Castro[1], Fernanda de Oliveira Mota[1], Mayara Satsuki Kunii[1], Maria de Los Angeles Duran Salas[1], Adriane da Silva Santos Ibanez[1], Cintia Monteiro Lustosa[1], Vanessa Aparecida do Nascimento Varjão[1], Camila Margarida Maria Soares de Sousa Parrode[1], Cristiane Menezes Vitória Alferi[1], Olga Margareth Felix[1], Adriana Seber[1,2]

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INTRODUCTION:

AML is a heterogeneous group of hematological diseases representing approximately 20% of acute leukemias in children. Unfortunately, 40% of them may die due to refractory or relapsed disease and treatment-related toxicities. HCT is a therapeutic option for these patients. Best results are associated with the disease phase at timing of transplant. Patients with refractory diseases have unfavorable outcomes and tend to be referred to exclusive palliative care. The use of HCT in patients with active AML remains controversial, but the pediatric BFM group (Sauer, 2019) has reported 41% 4-year event-free survival (EFS) in these patients using cytoreduction with FLAMSA prior to the HCT. Despite including few patients, this strategy has emerged to alter the tragic natural history of the refractory disease and has been adopted in the Brazilian AML protocol (GELMAI).

OBJECTIVE

To evaluate the outcomes of the allogeneic HCT performed for refractory AML.

CASUISTIC:

The patients in 21 out of 81 HCT performed from 2017 to 2024 for AML had refractory disease and were included in this study.

METHOD:

Refractoriness was defined as the presence of morphological > 5% blasts, unresponsive to chemotherapy. Conditioning regimens included Busulfan, Fludarabine, and Melphalan (Jaiswal et al.2016), Total Body Irradiation (TBI) and Fludarabine (Solomon et al.2019), and adapted FLAMSA (GELMAI). GVHD prophylaxis used PT-Cy, calcineurin inhibitor (CI) - MMF in haploidentical (Haplo), CI-Mtx in unrelated (MUD) and CI in matched sibling donor (MSD) HCT.

RESULTS:

A total 21 transplants for pediatric refractory AML were performed from 2017 to 2024 in 18 patients; three underwent a second HCT, two due to relapse and one due to primary graft failure. The median age was 8 years (1-17), 30% had FAB-M4 AML. Donors were 67% Haplo, 24% MUD, and 10% MSD. All but two HCT used myeloablative conditioning (MAC): 19% Bu-Flu-Mel, 10% Flu-TBI and 62% adapted FLAMSA. After FLAMSA cytoreduction, 7/13 patients received conditioning with Bu-CTX. 12/21 (57%), grafts were bone marrow (BM), and 43% peripheral blood stem cells (PBSC). Of the 13 patients who underwent adapted FLAMSA, 7 died: 3 due to relapse, 2 due to infection, 1 due to pulmonary hemorrhage and 1 of venoclusive disease; 6/13 patients are alive at a median follow up of 15 months. Six children received the scheduled prophylactic donor leukocyte infusion (DLI): two relapsed, one died of infectious complications and three remain in remission for over one year, only one with chronic GVHD. The overall survival is 40% at a median follow up of 16 months. Most deaths were due to relapse, highlighting the importance of post-HCT monitoring and prophylactic and preemptive maintenance regimens.

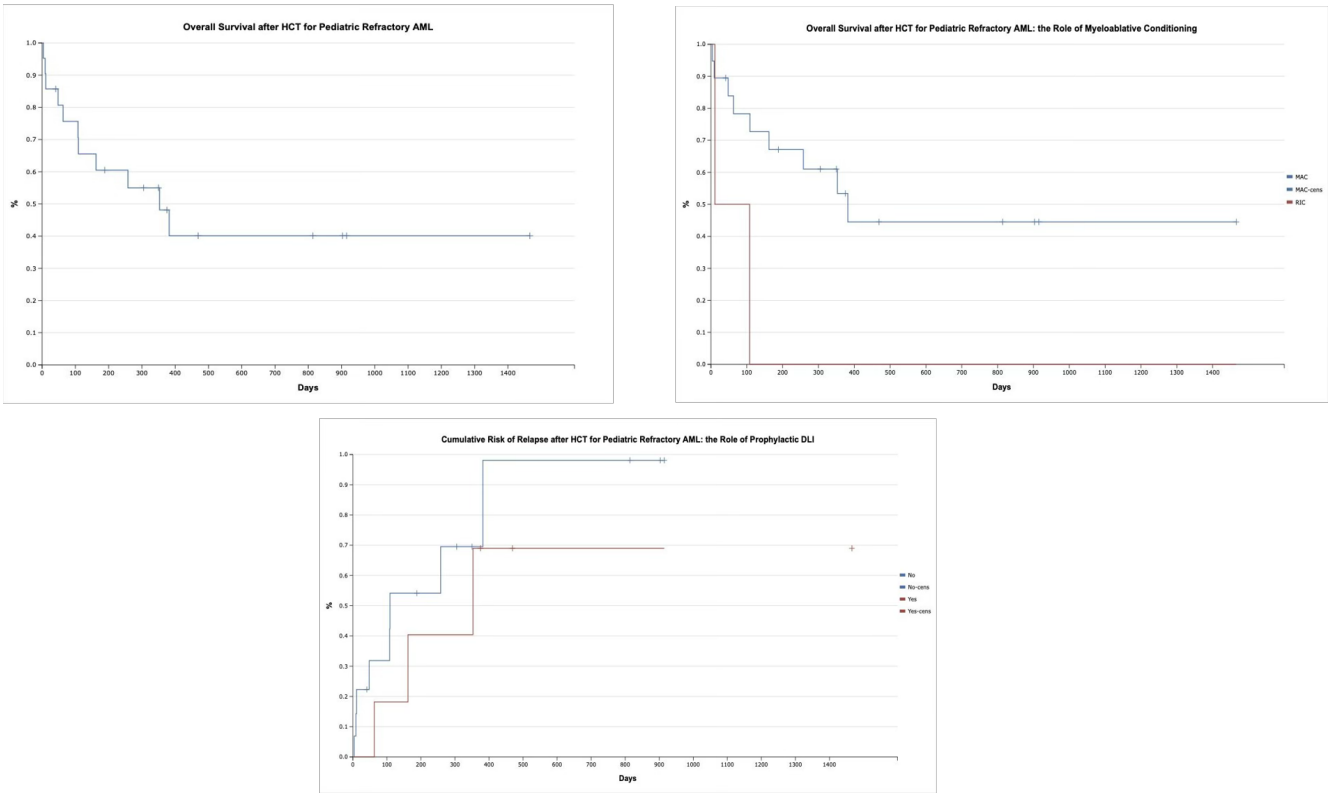
CONCLUSION:

HCT for patients with refractory/relapsed AML remains a challenge but the adapted FLAMSA regimen, myeloablative conditioning and prophylactic DLI are strategies that can offer a real chance of being cured of their disease.

FIGURE 1

Figure 1

TREATMENT OUTCOMES



ANALYSIS OF COMPLICATIONS IN PEDIATRIC RELATED DONORS UNDERGOING ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION AT A PHILANTHROPIC HOSPITAL IN SÃO PAULO

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INTRODUCTION:

Allogeneic bone marrow transplantation is an essential therapeutic option for children with severe hematological and oncological diseases, such as leukemia, congenital anemias, and immunodeficiencies. The selection of a child as a donor requires additional care and strict adherence to ethical, clinical, and legal criteria, considering their vulnerability and limited capacity for understanding and consent, in order to prevent further complications throughout the process.

OBJECTIVE:

To identify complications during the bone marrow donation process in pediatric patients. Case Series:

A cross-sectional study including 10 related pediatric donors, of both sexes, aged between 10 months and 16 years.

METHOD:

The bone marrow donation procedures took place between January 2023 and April 2025. Medical records and institutional data from a philanthropic hospital in São Paulo were analyzed. Collected data included demographic information, type of collection performed (bone marrow vs. peripheral blood), average time between transplant request and cell collection, and complications before, during, and after donation. Descriptive analysis was conducted using absolute and relative frequencies, and median values [minimum–maximum].

RESULTS:

The sample included 50% female donors. The median age was 10.5 years [0.83–16]. The median time between initial contact and collection was 2.5 months [1.7–10.6]. Regarding complications, three donors experienced events prior to the transplant: two with anxiety (ages 9 and 16) and one with vascular access issues, requiring six attempts (donor <1 year old). During collection, one patient experienced wheezing during intubation. After donation, 70% of donors presented complications, including mild pain (30%) and severe pain (10%). Other reported complications were hypotension (20%) and bleeding at the puncture site (20%).

CONCLUSION:

The analysis of complications revealed the presence of non-physical symptoms prior to transplantation, such as anxiety, which can result in a negative experience. Post-donation complications, including severe pain, hypotension, and bleeding at the puncture site, reinforce the importance of following well-structured protocols that emphasize prevention, emotional support, and early management. These findings highlight the need for careful monitoring to minimize risks and ensure donor safety at every stage of the process. Such actions not only protect the donor's health and well-being but also strengthen family trust throughout the donation journey.

KEYWORDS: Bone Marrow Transplantation; Pediatric Donors; Complications.

CHARACTERIZATION OF PEDIATRIC ONCOLOGY PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION IN A PUBLIC REFERRAL HOSPITAL IN BAHIA, BRAZIL

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT), involving the infusion of hematopoietic stem cells to replace diseased bone marrow following myeloablative chemotherapy, is a high-cost procedure. Despite this, over 100 centers across Brazil are equipped to perform HSCT. In Bahia, this treatment became a reality for pediatric patients under the Brazilian public health system (SUS) in 2020 at a regional referral hospital. Objectives: To characterize pediatric oncology patients who underwent HSCT from October 2020 to October 2024 at a SUS-affiliated referral hospital.

METHODOLOGY:

A retrospective cohort study through medical record review. Results: Among 32 HSCTs performed, 1 occurred in 2020, 9 in 2021, 6 in 2022, 8 in 2023, and 8 in 2024. 15 patients were female (47%) and 17 male (53%), aged between 3 and 19 years. Diagnoses included: neuroblastoma (59.4%), Hodgkin lymphoma (21.85%), non-Hodgkin lymphoma (6.25%), AML M3 (6.25%), and germ cell tumor (6.25%). Disease status pre-HSCT: 19 complete remission (59.4%), 9 partial remission (28.1%), 4 with active disease (12.5%). CD34+ cell dose ranged from 2.57 to 24.48 $\times 10^6$/kg (mean 5.73, median 4.73). Neutrophil engraftment occurred between 7–16 days (mean 10.96, median 11). Expected complications included mucositis and febrile neutropenia in all patients (100%). Post-HSCT maintenance therapy: Isotretinoin (46.8%), Anti-GD2 antibody (9.5%), Brentuximab (6.2%), Pembrolizumab (3.1%). No maintenance in 34.4% of cases. Radiotherapy post-HSCT: 14 patients (43.8%) underwent radiotherapy; 18 (56.2%) had no indication. Outcomes at Day +100: 29 patients in complete remission (90.7%), 1 with stable disease (3.1%), 1 relapse (3.1%), 1 death (3.1%) disease-related. Current status (as of October 2024): 20 in complete remission (62.5%), 12 with relapse (37.5%), of which 5 have active disease (41.7%) and 7 died (58.3%). Overall survival:

CONCLUSION:

In its first four years, the service performed over 30 autologous HSCTs, eliminating the state's transplant waiting list — a major public health achievement in Bahia. The data demonstrate that HSCT is an effective therapeutic strategy for achieving remission and improving prognosis, reinforcing its importance within the SUS at a regional referral center.

CLINICAL AND EPIDEMIOLOGICAL CHARACTERISTICS OF PEDIATRIC ALLOGENEIC TRANSPLANTS FOR MALIGNANT AND NON-MALIGNANT DISEASES: EXPERIENCE FROM A SINGLE CENTER

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INTRODUCTION:

Hematopoietic stem cell transplantation is essential in the treatment of malignant and non-malignant hematological diseases. Although it offers curative potential, success depends on controlling complications such as graft-versus-host disease (GVHD), infections, and hepatic sinusoidal obstruction syndrome (SOS). Factors such as HLA mismatch, age, and comorbidities increase the risk of mortality. Initially performed using bone marrow, stem cell collection has expanded since the 2000s to include peripheral blood mobilized by growth factors.

OBJECTIVE:

To describe the epidemiological profile and the most prevalent non-infectious complications in a pediatric bone marrow transplant service in the interior of São Paulo.

METHOD:

Retrospective analysis of medical records.

RESULTS:

Between November 2019 and May 2025, 102 patients underwent allogeneic transplantation. Of these, 24 (23.5%) with a matched related donor transplant, 16 (15.7%) with a matched unrelated donor transplant, and 62 (60.8%) with a haploidentical donor transplant. The median age at transplant was 9 years (range: 2 months–16 years), with 60 male children. Malignant diseases accounted for 82.3% of transplants (N=84). Conditioning regimens used were myeloablative in 87 patients (85.3%), non-myeloablative in 12 (11.8%), and reduced-intensity conditioning (RIC) in 3 (2.9%). Bone marrow was the graft source in 58.8% of transplants (N=60) and peripheral blood in 41.2% (N=42). Among the most prevalent non-infectious complications, acute GVHD grades III/IV occurred in 21.6% (N=22), chronic GVHD in 8.8% (N=9), SOS in 1.9% (N=2), thrombotic microangiopathy in 2.9% (N=3), and posterior reversible encephalopathy syndrome (PRES) in 0.98% (N=1). During the evaluated period, 20 patients (19.6%) experienced relapse of the underlying disease and 37 (36.2%) died. The overall survival in 5 years is 71,3% for non-malignant diseases and 57,8% for malignant diseases.

CONCLUSIONS:

There is a marked increase in haploidentical transplants. This strategy has become a viable alternative given the scarcity of fully matched donors, especially in pediatric contexts. The frequent choice of peripheral blood as the graft source reflects its association with a stronger graft-versus-leukemia (GVL) effect, potentially contributing to the control of malignant diseases, which comprised the majority of cases (82.3%). However, this benefit is counterbalanced by a higher incidence of severe immunological complications, particularly GVHD cases. These data reinforce the need for more effective prevention and management strategies for GVHD in pediatric transplants, aiming to improve long-term quality of life for these patients.

KEYWORDS: transplant; clinical characteristics; complications

DONOR LEUKOCYTE INFUSIONS MAY REVERT MIXED CHIMERISM AFTER ALLOGENEIC BONE MARROW TRANSPLANT FOR SICKLE CELL DISEASE AND PREVENT IMPENDING GRAFT REJECTION WITHOUT SEVERE GRAFT-VERSUS-HOST DISEASE (GVHD)

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INTRODUCTION:

Donor leukocyte infusions (DLI), commonly used after bone marrow transplant (BMT) for malignant diseases, is rarely used in non-malignant diseases due to the concern of inducing GVHD. However, decreasing chimerism may lead to graft rejection, and the need of a second BMT. Sickle cell disease (SCD), the most common hemoglobinopathy in Brazil, is curable by BMT. At least 35% donor cell chimerism is reported to be sufficient to avoid rejection in the HLA identical (MSD) setting, but in haploidentical BMT (Haplo), mixed chimerism (MC) frequently leads to graft failure. Increasing or decreasing immunosuppression to manage MC is a matter of debate. Our objective is to describe the use of low-dose DLI to reverse decreasing chimerism in patients with SCD.

CASUISTIC:

48 patients underwent 49 BMT for SCD; DLI was used in 4 of them, and in an additional patient that received haplo-HCT in another institution.

METHOD:

Retrospective analysis of all SCD patients after BMT that received DLI. Conditioning for Haplo was ATG 4,5mg/kg, Flu 150mg/m^2 and Cy 29mg/kg, TBI 200-400cGy and Thiotepa 10mg/kg in all but one patient and for BMT from MSD, ATG 6mg/kg, Bu 18mg/kg, Flu 150mg/m^2. GVHD prophylaxis was PT-Cy, sirolimus or tacrolimus + MMF in Haplo and CsA + MTX for MSD BMT. Serial chimerism was evaluated monthly by VNTR or STR. Fresh donor peripheral blood was used for DLI in all cases aiming a low dose of 5x105-5x10^6 CD3/kg, given every 30-90 days according to

the chimerism kinetics and stopped if chimerism was increasing or if the patient developed any GVHD. Data was collected from electronic medical records.

RESULTS:

From 2016 to 2025, we performed 49 BMT for SCD in 48 patients: 33 Haplo, 13 MSD and 3 MUD. Four of these patients received DLI due to mixed chimerism, as well as an additional patient that had BMT in another institution. The patients receiving DLI were 6-20 years of age, four had Haplo and one, MSD. DLI was considered when the chimerism was progressively decreasing to <70%. The mean day of DLI indication was D+163 (72-290). Patients received 2-9 DLIs, 5x105-5x10^6 CD3/kg. The reasons for interruption were stabilization or improvement of chimerism (4/5), development of GVHD (1/5) and one graft rejection, later undergoing a second BMT with complete donor engraftment. All donors had sickle cell trait and the HbS remained around 50%. One patient is still under evaluation and has a planned additional DLI due to chimerism of 82% in mononuclear cells. One important limitation is the lack of split chimerism availability in all patients.

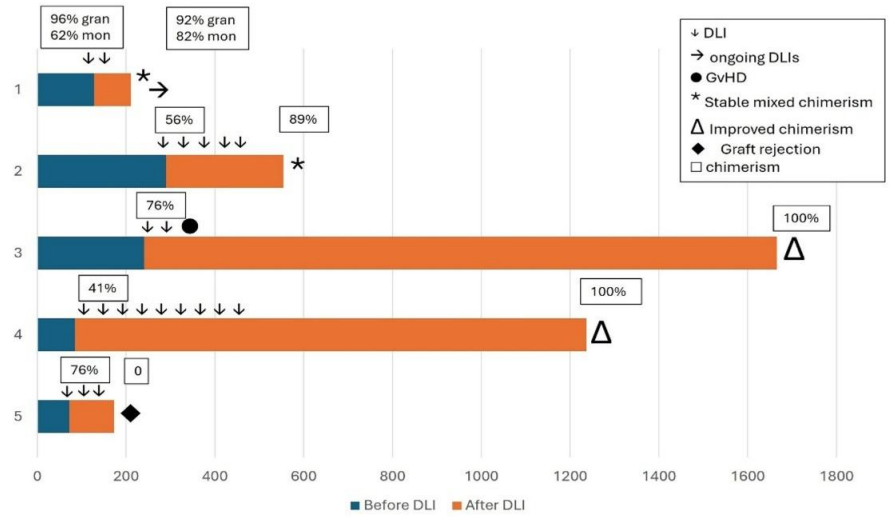
CONCLUSION:

Low dose DLI is a feasible and safe strategy to avoid rejection after BMT for sickle cell disease.

KEYWORDS: Sickle cell, bone marrow transplant, chimerism

FIGURE 1 – DLI to treat mixed chimerism in SCD

Patient	Gender	Age	Type of BMT	ABO patient	ABO donor	Conditioning regimen	GvHD prophylaxis	Time between BMT and DLI (days)	Chimerism before 1st DLI	Total DLIs	DLI dose range(x10⁶)	Immuno suppression during DLI (FK or Siro)	Last chimerism	GVHD	Outcome
1	M	6,6	Matched sibling donor	O	O	ATG Bu Flu	CSA + MTX	128	62% mon / 96% gran	2	1	Yes	82% mon / 92% gran	No	Stable, further DLI planned
2	M	10,5	Haplo – father	A	A	ATG TT Flu Cy TBI200	PT-Cy FK MMF	290	58%	5	0.5 - 5	No	88%	No	Stable mixed chimerism
3	F	14,2	Haplo – father	O	O	ATG Flu Cy TBI 400	PT-Cy Siro MMF	241	76%	2	0.9 - 1	Yes	100%	Chronic skin GVHD II	Improved chimerism
4	M	19,9	Haplo - sibling	A	A	ATG TT Flu Cy TBI200	PT-Cy Siro MMF	85	41%	9	0.1 - 5	Only in 1st DLI	100%	No	Improved chimerism
5	M	12	Haplo - mother	A	A	ATG TT Flu Cy TBI 200	PT-Cy Siro MMF	72	76%	3	1 - 6	Yes	0	No	Second BMT due to graft rejection



Legend: BMT: Bone marrow transplant; GVHD: graft versus host disease; DLI: Donor lymphocyte infusion; M: male; F: female; ATG: anti-thymocyte globulin; TT: thiotepa; Flu: Fludarabine ; Cy: Cyclophosphamide; TBI 200: Total body irradiation 200cGy; TBI 400: Total body irradiation 400cGy; PT-Cy: post-transplant Cyclophosphamide; FK: Tacrolimus; Siro: Sirolimus; MMF: Mycophenolate mofetil; mon: monocytes; gran: granulocytes.

HAPLOIDENTICAL HEMATOPOIETIC STEM CELL TRANSPLANTS IN PEDIATRIC PATIENTS WITH SEVERE APLASTIC ANEMIA OVER THE PAST DECADE

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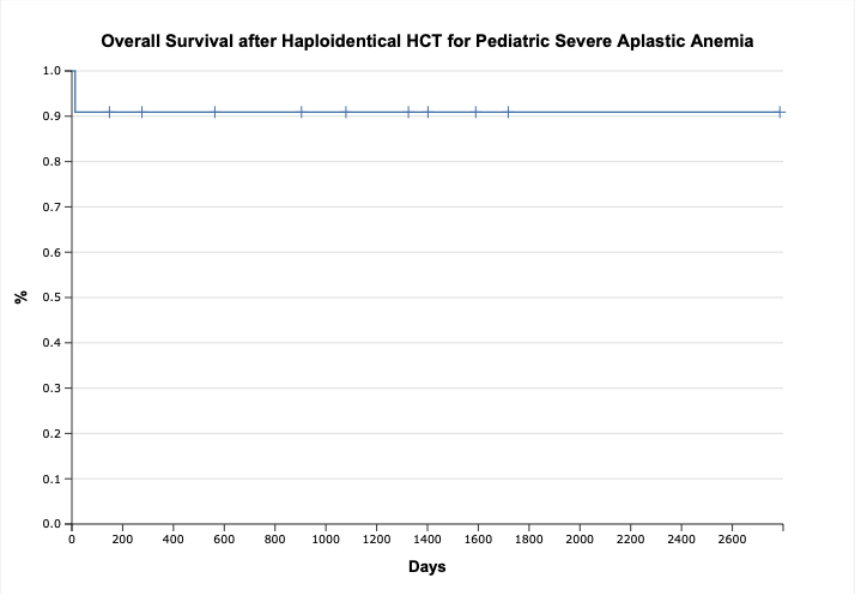
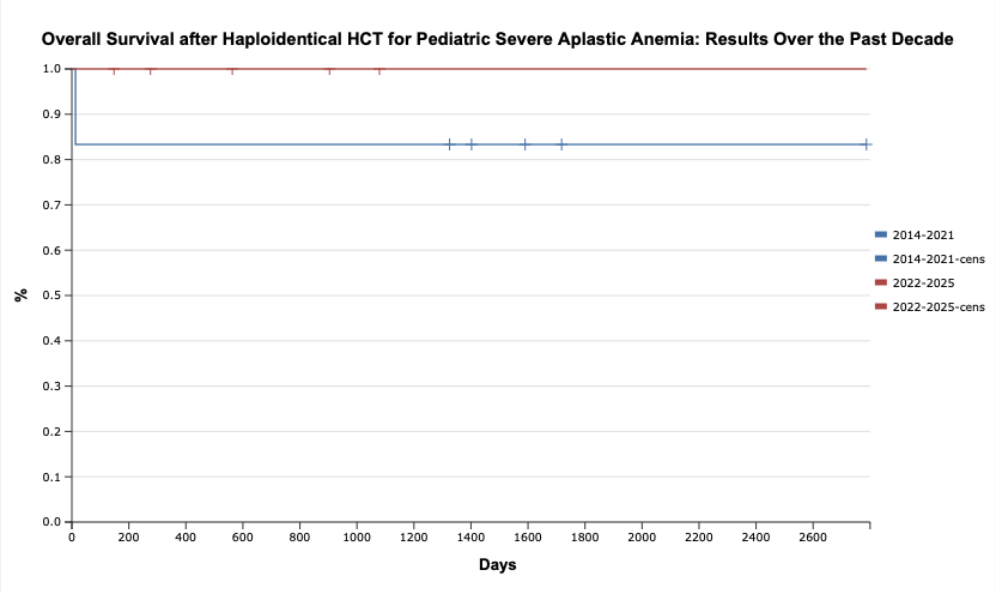
INTRODUCTION:

Severe aplastic anemia (SAA) is a serious and potentially fatal condition in children, and prompt hematopoietic stem cell transplantation (HCT) is considered the best therapeutic strategy. However, the absence of an HLA-identical donor poses a significant challenge, due to long waiting time from starting the search for an unrelated donor and the marrow collection for the HCT. Haploidentical HCT (Haplo) is a promising alternative, but the process of selecting the best donors, avoiding mothers, those with more immunogenic non-permissive mismatches or with HLA typing against which the patient has the presence of antibodies has been only recently learned. Since we have performed Haplo for SAA for over a decade and so much have changed, our objective is to evaluate treatment outcomes over time to have up to date data to better counsel the families of children with SAA and no matched sibling donor available. Casuistic included 11 pediatric patients diagnosed with SAA, who underwent Haplo between 2014 and 2025. Method: Review of electronic medical records to define conditioning, GVHD prophylaxis, stem cell source, engraftment, severe infections, transplant-related toxicities, acute and chronic graft-versus-host disease (GVHD), and overall survival defined by the Kaplan-Meier method. Results: The median age was 8 years (4-14), 82% males. Bone marrow was the stem cell graft in all patients but the first two, who underwent Haplo as a second transplant after graft failure of a first non-Haplo HCT. Conditioning with 400cGy TBI was used in all first transplants, and ATG in 64%. For graft-versus-host (GVHD) prophylaxis, all patients received post-transplant cyclophosphamide (PT-Cy), mycophenolate mofetil (MMF) and calcineurin inhibitor. Donors were father (8), mother (2) and aunt (1). ABO incompatibility was present in half of the HCT (27% major and 18% minor). The first was the only patient that has died on D+13 after non-engraftment of a first unrelated donor HCT, and the immediate cause of death was venoocclusive disease. With a median follow up of 3.2 years, overall survival is 91%: 83% 2014-2021 and 100% 2022-2025 (Logrank p=0.36). Seventy percent of the children had viral reactivations: 64% CMV, 27% herpes zoster, 27% BK viral cystitis. Reversible posterior encephalopathy syndrome was diagnosed in two patients and sinusoidal obstruction syndrome

in one. Acute and chronic GVHD were observed in 64% and 36% of the patients, respectively, with no severe forms. There was no graft failures in these cohorts. Conclusions: Haploidentical HCT proved to be a viable and safe alternative for the treatment of children with SAA without an HLA matched donor, with high overall survival and manageable toxicities but viral reactivations are very frequent and deserve rigorous follow up and preemptive treatment strategies. A prospective multicenter study is being performed to evaluate the HCT strategies in our country.





HLA-IDENTICAL HEMATOPOIETIC STEM CELL TRANSPLANTATION (HCT) IN CHILDREN WITH HEMATOLOGIC MALIGNANCIES: CHALLENGES AND OUTCOMES

Luciana dos Santos Domingues[1]; Ana Carolina Ribeiro Correa[1]; Laís Lima Quintino[1]; Maria Gabriela Alves Dias Matos[1]; Raisa Machado Amaral[1]; Valéria Cortez Ginani[2]; Roseane Vasconcelos Gouveia[2]; Érica Almeida Viana[1]; Ana Cláudia Ramos Donatelli Bronzoni[1]; Aline Ferrari Martins[1]; Fernanda de Oliveira Mota[1]; Yohanna Bastani de Mattos[1]; Lahis Cardoso de Castro[1]; Maria de Los Angeles Duran Salas[1]; Maria Estela Shiroma[1]; Mayara Satsuki Kunii[1]; Nathalia Gonçalves Rissardi1; Adriane da Silva Santos Ibanez[1]; Cintia Monteiro Lustosa[1]; Camilla Margarida Maria Soares de Sousa Parrode[1]; Vanessa Aparecida do Nascimento Varjao[1]; Michael Jenwei Chen[1]; Maria Luísa Sucharski Figueiredo[1]; Olga Margareth Felix[1]; Dafne Cardoso da Silva[1]; Cristiane Menezes Vitoria Alferi[1]; Henrique Manoel Lederman[1]; Alberto Cardoso Martins Lima[3]; Gisele Fabianne Rampim[3]; Isaú Henrique Noronha[3]; Tuila Bittencourt Mourao[3]; Adriana Seber[2].

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FIGURE 1

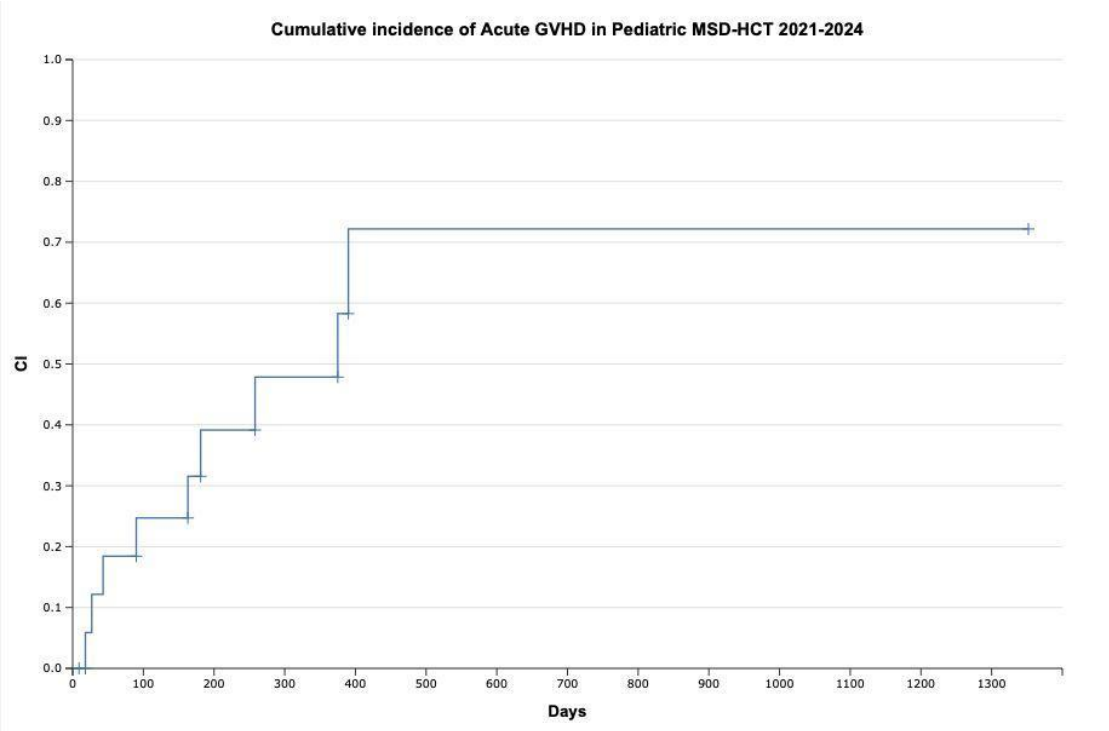


FIGURE 2

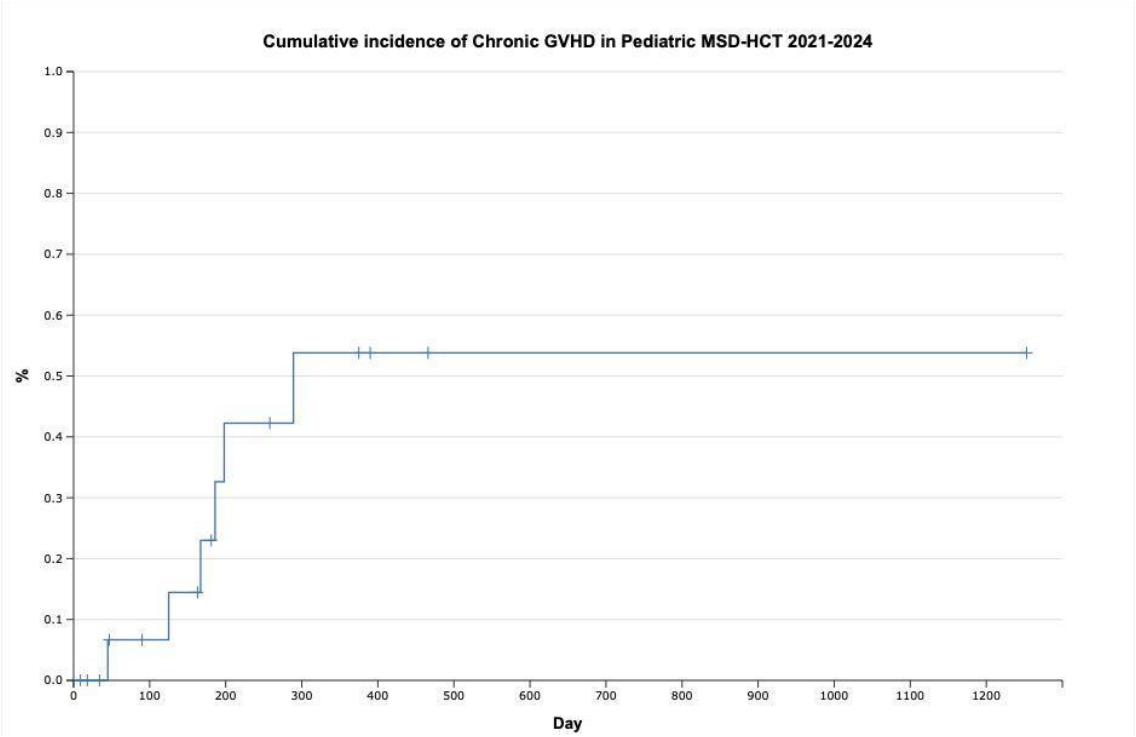
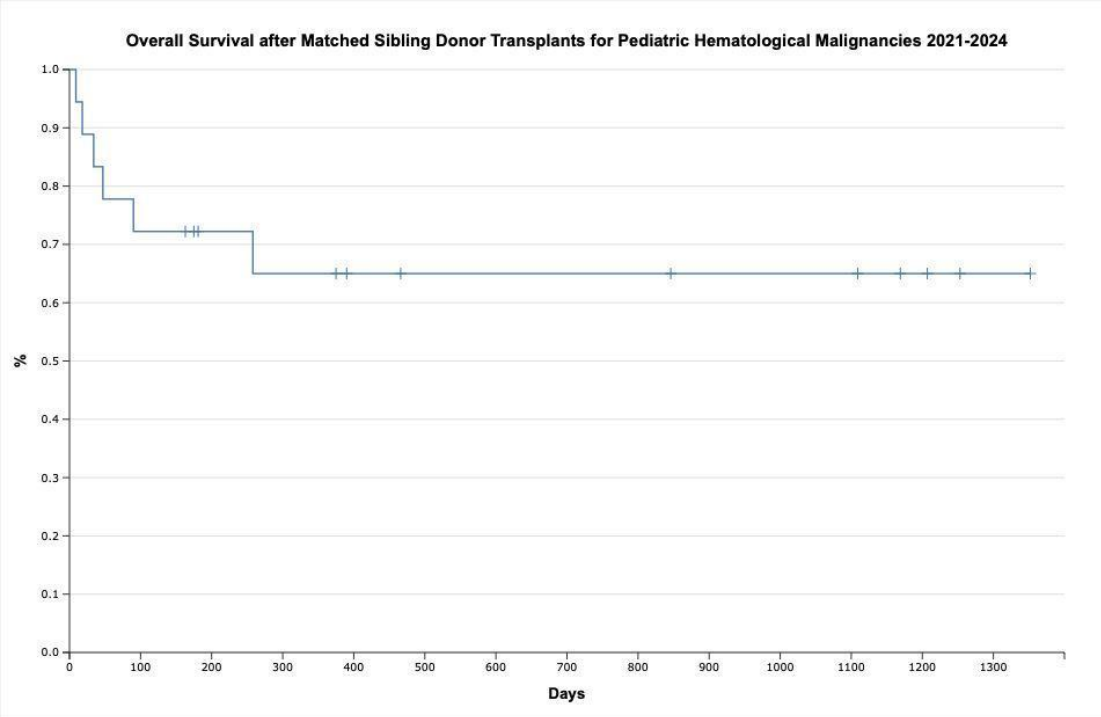


FIGURE 3



HUMANIZATION STRATEGY FOR PEDIATRIC PATIENTS UNDERGOING TOTAL BODY IRRADIATION IN BONE MARROW TRANSPLANTATION PREPARATION

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Total Body Irradiation (TBI) is a crucial step in the preparatory regimen for bone marrow transplantation, aimed at achieving complete immunosuppression. However, due to its nature—prolonged exposure of the entire body to radiation in a large, cold, and isolated room—TBI poses unique emotional and physical challenges, particularly for pediatric patients. During each session, which lasts an average of 17 minutes per side in lateral or dorsal decubitus, children remain alone for technical and safety reasons. This isolation, coupled with the physical discomfort, often generates fear, distress, and a sense of abandonment. In response to this, a humanization intervention was implemented with the goal of improving the experience of pediatric patients during TBI. The intervention involved the strategic placement of a projector within the irradiation room to display personalized audiovisual content such as cartoons, superhero videos, or other child-selected themes. These preferences were identified during a preparatory psychological evaluation, in which a psychologist assessed the child's emotional needs and coping strategies. This approach proved highly effective in reducing anxiety, promoting positive distraction, and increasing patient cooperation during the procedure. Furthermore, the strategy strengthened the bond between the multidisciplinary healthcare team and the patient, creating a more welcoming and supportive environment in an otherwise highly technical context. The results underscore the importance of incorporating humanization practices in radiotherapy settings, particularly in high-complexity pediatric cases. This intervention highlights how psychological and emotional support can be integrated into medical procedures to enhance overall patient care and experience.

KEYWORDS:

Pediatric Psychology; Humanization; Total Body Irradiation

"I'M GOING TO CHANGE MY LITTLE MARROW, NOW WHAT?": DEVELOPMENT OF AN INTERACTIVE THERAPEUTIC BOOK FOR CHILDREN UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION

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Hematopoietic Stem Cell Transplantation (HSCT) in children is a complex medical procedure involving extended hospitalization, aggressive treatments, and significant emotional challenges. To support children's psychological understanding and emotional coping, an interdisciplinary team developed an interactive therapeutic book titled *"I'm Going to Change My Little Marrow, Now What? A Journey of Courage and Hope."* This tool integrates instructional and dramatic therapeutic play with educational and interactive components. Interdisciplinary meetings were held to identify pediatric patients' needs regarding diagnosis comprehension, medical procedures, and treatment stages. A script was created and validated by health professionals, resulting in a 20-page book covering hospital admission, diagnosis, laboratory and imaging tests, physical examinations, medical devices, treatment phases (chemotherapy, radiotherapy, infusion, engraftment), side effects, multidisciplinary care, post-discharge instructions, and the child's role in their own care. Currently in use, the book is well accepted by patients and families. As an instructional therapeutic toy, it prepares children for procedures; as a dramatic play tool, it fosters emotional expression; as an interactive instrument, it stimulates creativity and engagement; and as an educational resource, it explains treatment steps through playful language. The structured use of therapeutic play promotes communication, reduces anxiety, and helps children process their hospital experience. The book has proven to be an essential tool for promoting understanding and reducing stress in children undergoing HSCT.

KEYWORDS:

Hematopoietic Stem Cell Transplantation; Pediatric Psychology; Therapeutic Play

IS IT SAFE TO TRANSPLANT MISMATCHED UNRELATED DONORS GUIDED BY AN OUTSTANDING HLA SPECIALIZED TEAM AND USING POST-TRANSPLANT CYCLOPHOSPHAMIDE

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INTRODUCTION:

Hematopoietic stem cell transplantation (HCT) is a well-established therapy for several malignant and non-malignant hematological diseases. The absence of a fully compatible donor is a frequent obstacle, especially in populations with high genetic diversity. Unrelated mismatched transplants (MMUD) have historically been associated with a higher risk of complications, poorer overall survival, increased risk of graft-versus-host disease (GVHD), and non-relapse mortality. However, the use of post-transplant cyclophosphamide (PTCy) as prophylaxis has modified this scenario, allowing greater safety in the use of alternative donors.

The objective of this study is to evaluate the incidence and severity of graft-versus-host disease (GVHD) in pediatric patients undergoing HCT with mismatched unrelated donors using PT-Cy as GVHD prophylaxis. Casuistic: 18 patients under the age of 18 years who underwent HCT between September 2022 and April 2025.

METHOD:

This is a retrospective study that used electronic medica records and the HCT service database. The best allogeneic donors are selected in weekly meetings with the IGEN immunogenetic team. All donors were younger than 35 years of age. GVHD prophylaxis consisted of cyclophosphamide 50mg/kg on D+3 and D+4, and mycophenolate mofetil and cyclosporine from D+5 onwards.

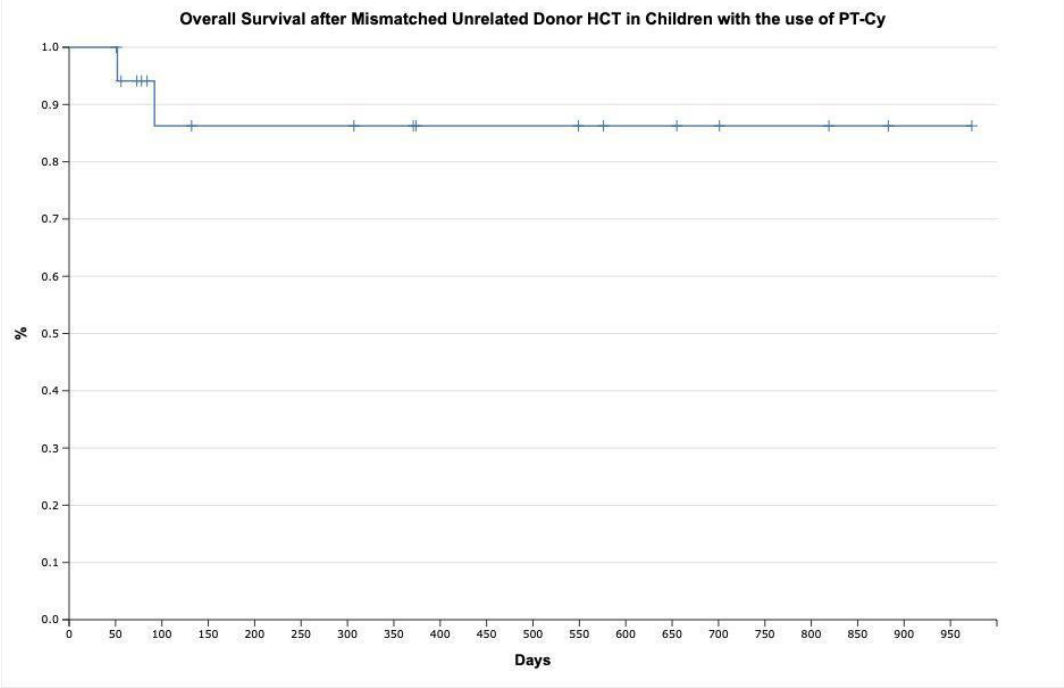
RESULTS:

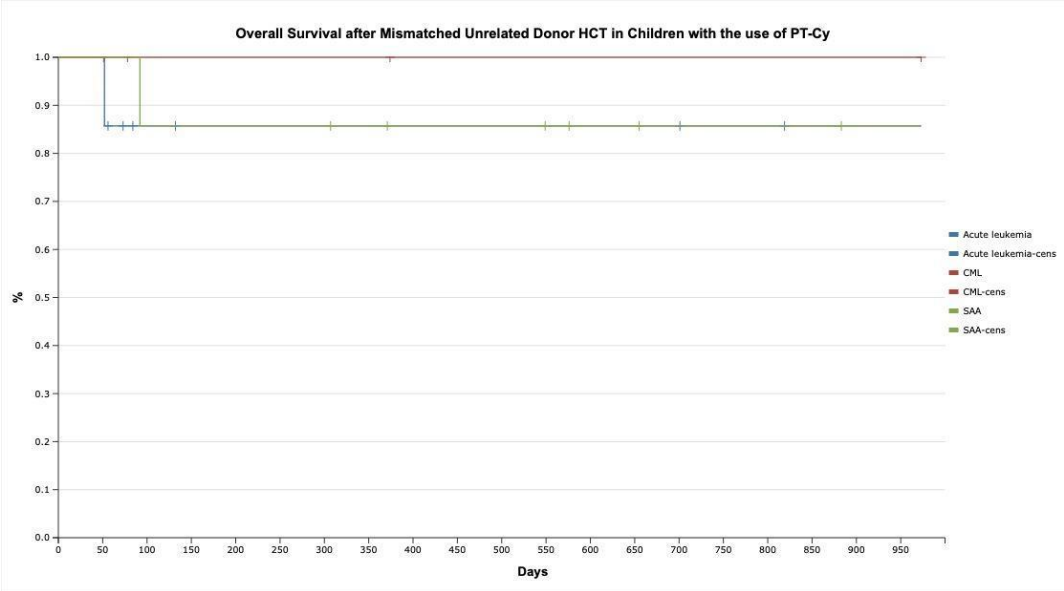
The 18 patients had severe aplastic anemia (7), chronic myeloid leukemia (4), acute lymphoblastic leukemia (6), and acute myeloid leukemia (1). The

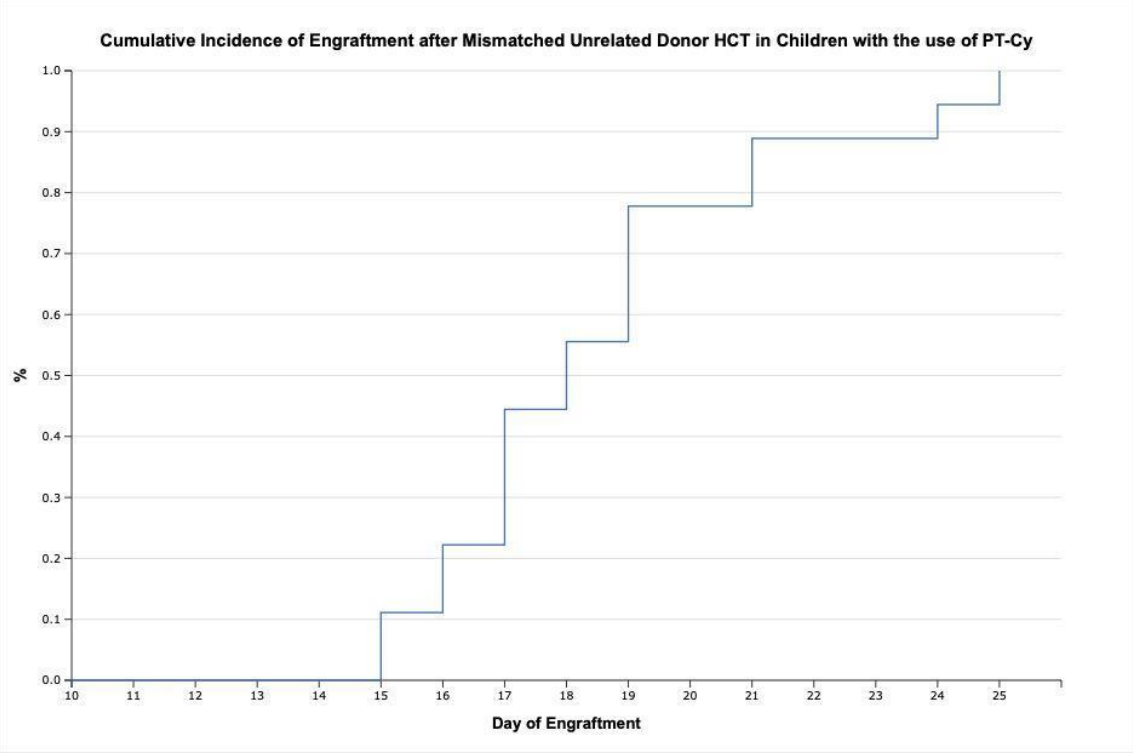
graft was bone marrow in 12 and peripheral blood in 6 HCT. Conditioning was myeloablative (MAC) in 14 patients and reduced-intensity (RIC) in 4. Patients with non-malignant diseases received additional serum therapy (ATG n = 7; Alemtuzumab n = 1). All patients engrafted after a median of 18 days. The incidence of acute GVHD was 16.7% (3/18), 3 classified as MAGIC III. The incidence of chronic GVHD was 6.2% (1/16), although follow-up is still short. There were two deaths before D+100 due to sepsis (1) and pulmonary fibrosis (1). Most patients reactivated CMV (61%). There were no deaths related to GVHD.

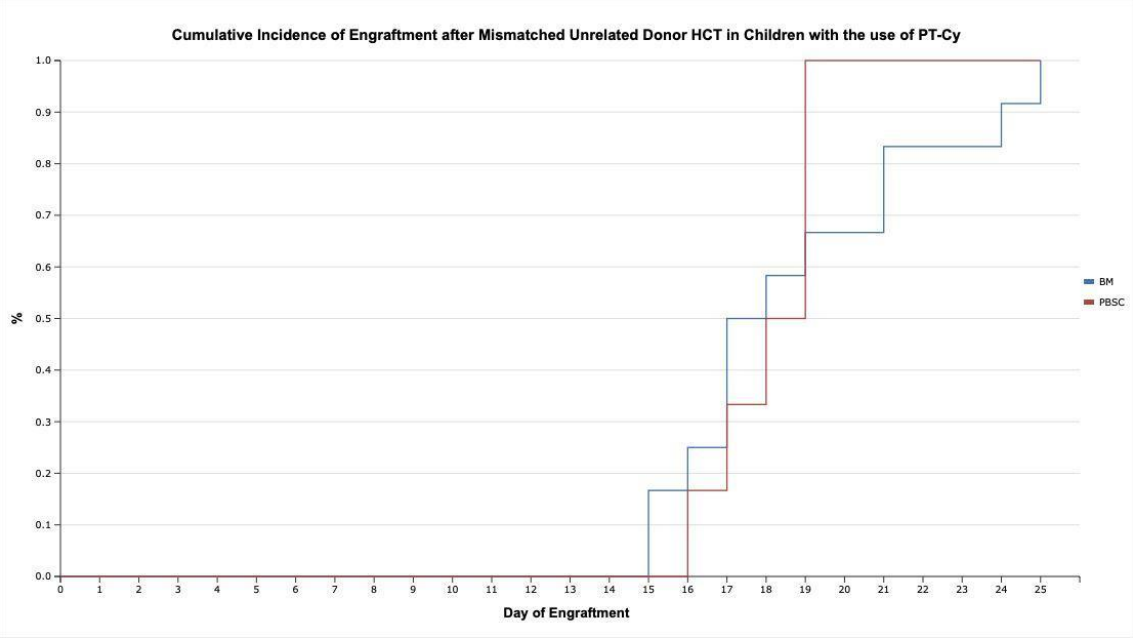
CONCLUSION:

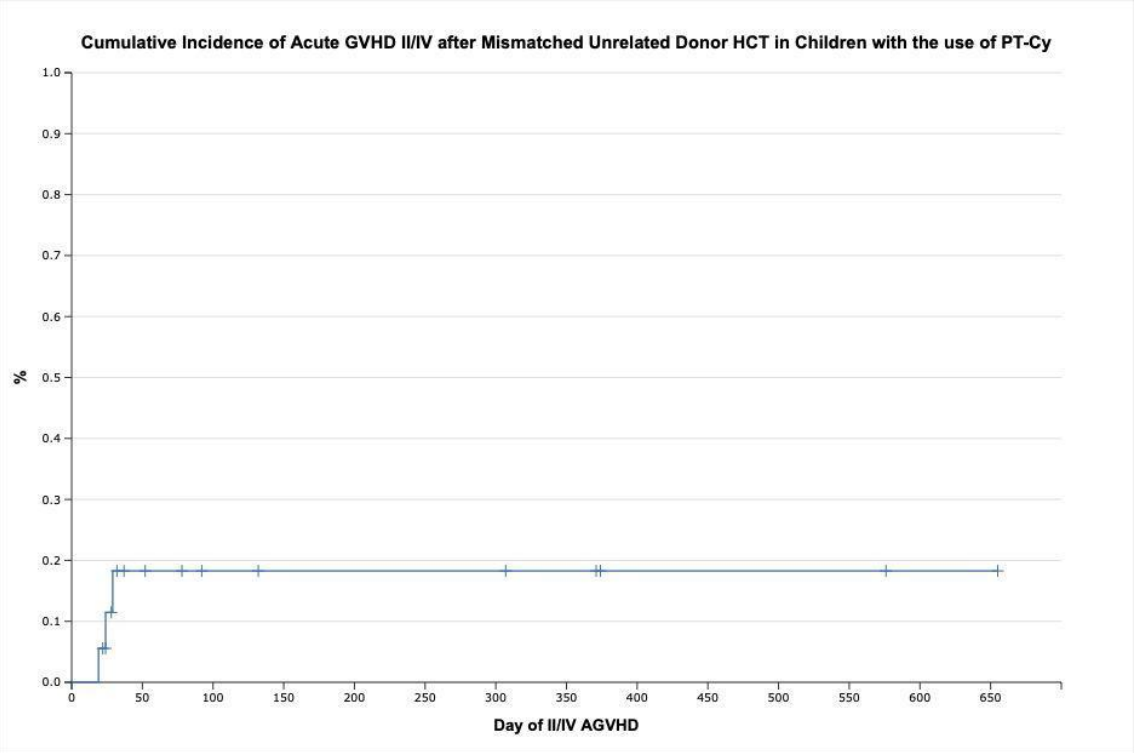
PTCy prophylaxis in pediatric HCT from MMUD is safe, with a low incidence of acute and chronic GVHD, successful engraftment, and low transplant-associated mortality. These findings support the use of MMUD with PTCy as a viable strategy to expand access to HCT.

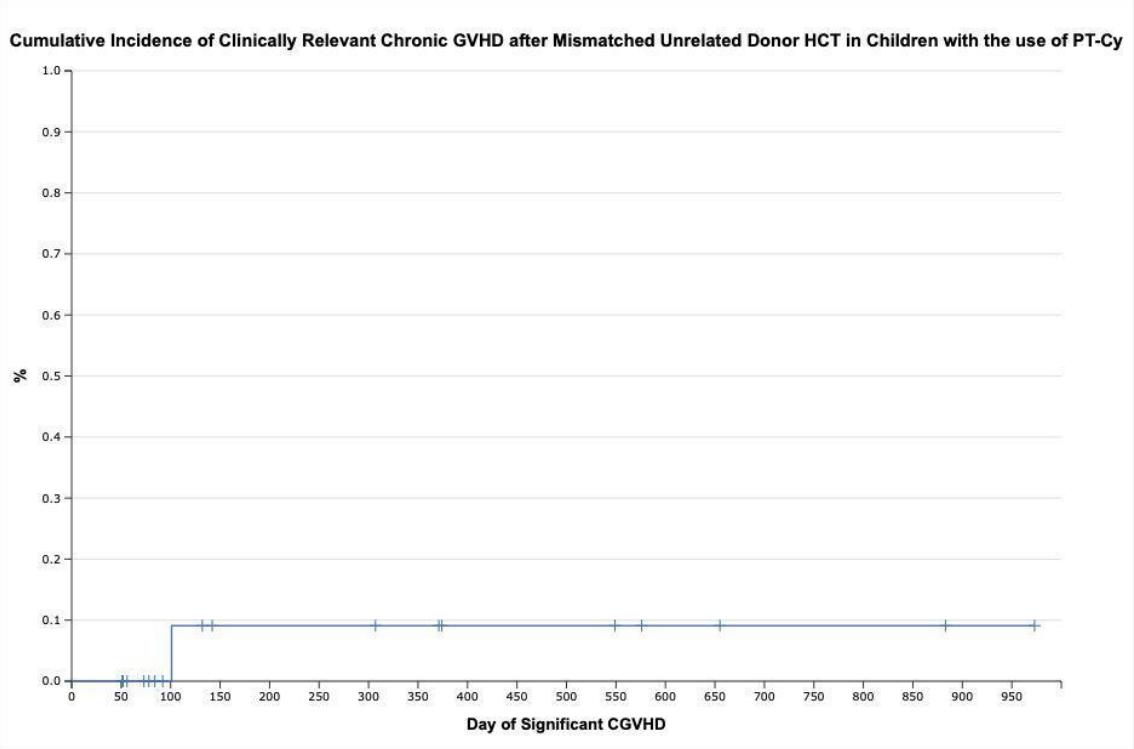





Cumulative Incidence of Engraftment after Mismatched Unrelated Donor HCT in Children with the use of PT-Cy
%
Day of Engraftment


Cumulative Incidence of Engraftment after Mismatched Unrelated Donor HCT in Children with the use of PT-Cy
%
Day of Engraftment
BM
PBSC


Cumulative Incidence of Acute GVHD II/IV after Mismatched Unrelated Donor HCT in Children with the use of PT-Cy
%
1.0
0.9
0.8
0.7
0.6
0.5
0.4
0.3
0.2
0.1
0.0
0
50
100
150
200
250
300
350
400
450
500
550
600
650
Day of II/IV AGVHD


Cumulative Incidence of Clinically Relevant Chronic GVHD after Mismatched Unrelated Donor HCT in Children with the use of PT-Cy
%
1.0
0.9
0.8
0.7
0.6
0.5
0.4
0.3
0.2
0.1
0.0
0
50
100
150
200
250
300
350
400
450
500
550
600
650
700
750
800
850
900
950
Day of Significant CGVHD

LOW-LEVEL DONOR-SPECIFIC HLA ANTIBODIES DO NOT IMPACT ENGRAFTMENT AFTER PEDIATRIC HEMATOPOIETIC CELL TRANSPLANTATION: A CASE SERIES

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2. IGEN/AFIP, São Paulo - SP - Brasil;
3. GRAAC & Hospital Samaritano, São Paulo - SP - Brasil;
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INTRODUCTION:

It is currently unknown whether low-level donor-specific HLA antibodies (DSA) affect engraftment in pediatric patients undergoing hematopoietic cell transplantation (HCT).

OBJECTIVE:

Thus, we present a case series evaluating low-level DSAs before and after transplant in a reference pediatric HCT unit.

METHODS:

Low-level DSAs were defined as those that are positive (MFI>300) in the Single Antigen Bead (SAB) assay but negative in Flow Cytometry Crossmatch (FCXM). DSA assessment was conducted using One Lambda's SAB assay. FCXM was performed using the GoHalifaster protocol. Desensitization strategies were tailored to the patient's disease and clinical condition. SAB testing was repeated on day zero (graft infusion) and day +2 to monitor potential DSA rebound. Chimerism analysis was performed using STR method.

RESULTS:

From 2022 to 2025, six pediatric patients were closely monitored for low-level DSAs. Details on the patient's diagnosis, desensitization, MFI values pre- and post-HCT, and HCT outcomes are provided in Figure 1A. In brief, patients 1 to 4 had low-level DSAs with negative FCXM results. After desensitization, all DSAs were below 1000 MFI on day zero, and no DSA rebounds were observed on day +2. All four patients achieved hematologic recovery, with complete chimerism on day +28. Patient 5, who had a Wiskott-Aldrich syndrome, exhibited an anti-DR7 DSA (MFI=3820), which was explained by 98E eplet. This DSA displayed an overlapping low-level pan-DR reactivity (Figure 1B), and the FCXM yielded a clear negative result. Despite this unusual DSA pattern,

the HCT team performed desensitization, and the patient fully engrafted. Patient 6 had a 10/10 matched unrelated donor with DP3 and DP14 mismatches, classified as nonpermissive in the host-versus-graft direction. We noted a 56ED eplet pattern just below 300 MFI, with DP3 and DP14 clustering with DP6, DP9, DP17, and DP20 (Figure 1C). Owing to the risk of graft failure due to nonpermissive DPB1 status, we monitored for 56ED DSA rebound, and no increase in DP3/DP14 reactivity was observed. Patient 6 achieved hematologic recovery, with complete engraftment on day +28.

CONCLUSION:

Our case series suggests that low-level DSAs, promptly desensitized, with negative FCXM and without DSA rebound post-HCT, are permissive for engraftment after HCT. Independent validation of our findings in the pediatric HCT setting is warranted.

FIGURE 1A) Patient, donor and DSA characteristics and HCT outcomes

Patient number	Diagnosis	Donor type	Desensitization protocol	Number of DSAs	DSA specificity	MFI before desenstitization	MFI after desensitization	MFI on day zero	MFI on day +2	Neutrophil recovery	Platelet recovery	Chimerism on day +28	Last Chimerism	Survival Status	Last Follow-Up
1	ALL	Haploidentical	MD Anderson[#]	1	DQ7 (DQA1*05:05/ DQB1*03:01)	2807	408	942	672	Day +16	Day +21	100% donor	100% donor	Deceased with sustained engraftment due to hepatic GVHD	Day +332
2	SCD	Haploidentical	Vanderbilt[†]	1	B*35:01	2322	359	521	736	Day +16	Day +23	100% donor	100% donor	Alive	Day +748
3	SAA	Haploidentical	MD Anderson[#]	2	DRB1*09:01	1831	400	925	783	Day +14	Day +19	100% donor	95.5% donor	Alive	Day +194
					DQ9 (DQA1*03:02/ DQB1*03:03)	2598	571	658	785						
4	SCD	Haploidentical	Vanderbilt[†]	1	B*07:02	1300	478	761	31	Day +18	Day +20	100% donor	97% donor	Alive	Day +793
5	WAS	Haploidentical	Three session of plasmapheresis	1	DRB1*07:01	3820	859	NT	NT	Day +18	Day +20	100% donor	100% donor	Alive	Day +121
6	SAA secondary to PNH	10/10 MUD with 2 DPB1 mismatches	One session of plasmapheresis for PTR due to strong class I HLA antibodies	2	DPB1*03:01	NT	NT	270	329	Day +19	Day +21	100% donor	100% donor	Alive	Day +258
					DPB1*14:01	NT	NT	170	150						

Abbreviations: ALL, Acute lymphoblastic leukemia; SCD, Sickle cell disease; SAA, Severe aplastic anemia; WAS, Wiskott-Aldrich syndrome; PNH, Paroxysmal nocturnal hemoglobinuria; MUD, matched unrelated donor; PTR, Platelet transfusion refractoriness; DSA, donor-specific anti-HLA antibodies; MFI, mean fluorescence intensity; NT, not tested; GVHD, graft-versus-host disease.

[#]**MD Anderson protocol:** Alternate day plasma exchange, followed by 1 dose of 375 mg/m2 rituximab (Rituxan) and 1 dose of intravenous immunoglobulin (IVIG) 1 g/kg and donor buffy coat.

[†]**Vanderbilt protocol:** Alternate-day, single-volume plasmapheresis with IVIg (100 mg/kg), tacrolimus (1 mg, IV per day) and mycophenolate mofetil (1 g, twice daily).

FIGURE 1B) DSA pattern of patient #5 (98E eplet reactivity overlapping with pan-DR pattern)

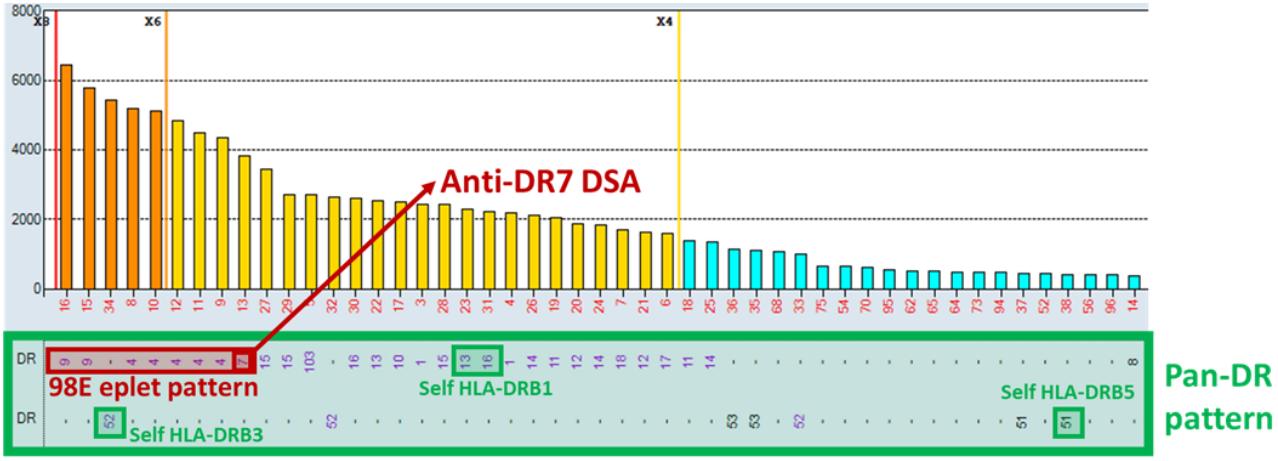
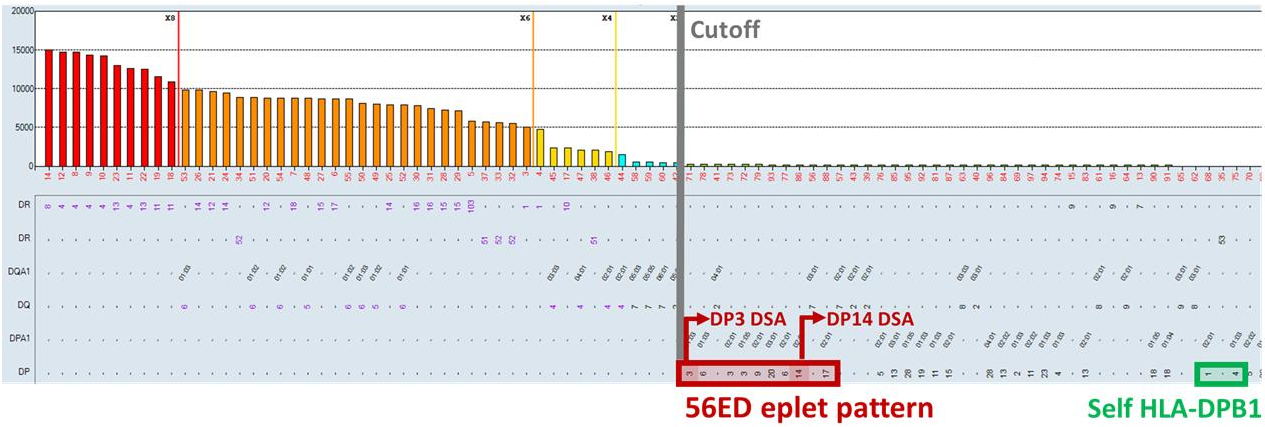


FIGURE 1C) **DSA pattern of patient #6 (56ED eplet reactivity just below the 300 MFI cutoff)**



NEXT-GENERATION SEQUENCING AS A DECISIVE TOOL FOR THERAPEUTIC GUIDANCE IN B-CELL ACUTE LYMPHOBLASTIC LEUKEMIA WITH ETV6::RUNX1: A RELAPSE CASE ASSOCIATED WITH RUNX1::CTC1 FUSION

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INTRODUCTION:

B-cell acute lymphoblastic leukemia (B-ALL) is the most common malignant neoplasm in childhood. About 25% of cases harbor the ETV6::RUNX1 fusion, classically associated with good prognosis. However, studies report a 20–24% relapse rate in patients with this translocation, raising doubts about its prognostic value.

OBJECTIVE:

To identify additional molecular alterations, by analyzing diagnostic and relapse samples from a B-ALL patient with ETV6::RUNX1 rearrangement, using next-generation sequencing (NGS) to investigate the probable factors involved in the relapse.

METHOD:

RNA sequencing was performed using the TruSight RNA Fusion panel (Illumina®). NGS workflow included library preparation, quality control, sequencing, and data analysis to assess gene expression, gene fusions, and genetic variants.

RESULTS:

A female patient, 26 months old, diagnosed with B-ALL, leukocytes 14,000, 55% blasts in peripheral blood, and 74% in bone marrow. Karyotype: 46,XX and ETV6::RUNX1 fusion. She was treated according to BFM 2009 protocol, with good initial response (minimal residual disease (MRD): 0.11% on D15; 0.006% on D33; undetectable on D78), and classified as intermediate risk. On the 90th week of treatment, she presented leukocytosis (18,330), blasts in peripheral blood, and central nervous system infiltration. Bone marrow confirmed medullary relapse (1.98 years after diagnosis). She was then treated with BFM 2022 protocol, without response. Blinatumomab was initiated, also with no response (MRD = 84.7%). Subsequently, a protocol with carfilzomib was initiated, reducing MRD to 0.03%. A new cycle of blinatumomab cleared MRD, enabling a haploidentical allogeneic transplant 10 months after relapse. She developed cutaneous graft-versus-host disease, clinically controlled. To date, the patient remains in morphological remission. NGS analysis identified, in addition to the recurrent ETV6::RUNX1 translocation, the RUNX1::CTC1 fusion in both diagnostic and relapse samples.

CONCLUSION:

Despite the ETV6::RUNX1 rearrangement, classically associated with favorable prognosis in B-ALL, the patient experienced relapse and treatment resistance. NGS analysis revealed the RUNX1::CTC1 fusion. RUNX1 has been implicated in fusions with over 55 different partner genes, contributing to

various leukemogenic mechanisms. CTC1 gene is involved in telomere maintenance, genomic stability, and processes such as DNA replication and repair. Telomere dysfunction, a known marker of chromosomal instability, may account for the observed clinical aggressiveness and treatment resistance, and may hold value as a prognostic biomarker in B-ALL. While RUNX1 fusions are critical in pediatric B-ALL, the presence of other genetic alterations can complicate the disease landscape, suggesting a need for comprehensive genomic profiling in affected patients. These findings highlight the value of NGS as a complementary diagnostic tool, enabling more accurate risk stratification and more effective therapeutic decisions from the outset.

KEYWORDS:

B-cell acute lymphoblastic leukemia; ETV6::RUNX1 fusion; Next-generation sequencing (NGS).

FUNDERS:

Foundation for research support of the Federal District (FAP-DF).

ROMIPLOSTIM IN HEMATOPOIETIC STEM CELL TRANSPLANTS WITH PLATELET TRANSFUSION RESTRICTIONS

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INTRODUCTION:

Patients undergoing hematopoietic stem cell transplantation (HCT) are expected to have severe thrombocytopenia. We have been through several blood shortages e.g. holidays, viral outbreaks, live mass vaccinations, environmental disasters and lack of public education to increase volunteer donations. Some patients refuse transfusions due to religious convictions, such as the Jehovah's Witnesses. In the latter, hematologic support may be limited, potentially compromising the safety of the procedure. Romiplostim, a thrombopoietin receptor agonist, has shown promise in refractory thrombocytopenia scenarios, but its use in the HCT setting remains underexplored. Our objective was to investigate the role and safety of romiplostim as an adjuvant agent for platelet support in pediatric HCT to reduce platelet transfusion requirement, prevent adverse effects and alloimmunization, and also include patients with transfusion objection based on religious beliefs.

METHOD:

Romiplostim was administered at 5 µg/kg/week subcutaneous starting on day +2, and continued until hematologic recovery.

RESULTS:

Our first patient was a 6 year-old girl diagnosed with stage IV neuroblastoma, previously treated with multiple therapeutic lines and subsequently referred for an HLA-identical related myeloablative HCT with busulfan-melphalan, with restriction to blood transfusions due to religious reasons. With the use of Romiplostim she maintained platelet counts above the critical levels of 10,000/mm^3 for most of the HCT course. She received only one platelet transfusion on day +10 due to a lower platelet count, fever and sepsis, from which she completely recovered. Neutrophil engraftment was achieved on day +10 and platelet engraftment on day +17. No severe hemorrhagic events or romiplostim-related toxicities were observed.

CONCLUSION:

Romiplostim proved to be a safe and effective alternative for managing thrombocytopenia in this first pediatric HCT patient with transfusion restrictions. This experience broadens the potential use of the agent, suggesting its applicability as a personalized therapeutic strategy in the HCT setting. Its use may be considered not only in cases of religious objection, but also as an approach to minimize transfusions and prevent alloimmunization. Further studies are needed to validate its efficacy on a larger scale, define cost-efficiency and support its integration into clinical practice.

KEYWORDS: Pediatric HSCT, Transfusion restriction, Romiplostim

SECONDARY GRAFT FAILURE DUE TO REFRACTORY CYTOMEGALOVIRUS AFTER HAPLOIDENTICAL TRANSPLANT IN A CHILD WITH SEVERE APLASTIC ANEMIA

Carla Nolasco Monteiro Breviglieri[1], Camila Noronha Santos1, Roseane Vasconcelos Gouveia[1,2], Valéria Cortez Ginani[1,2], Anna Beatriz Willemes Batalha[1], Milena Reis Santos de Oliveira[1,3], Márcia Puato Vieira Pupim[1], Gabriella Sayuri de Alencar[1], Juliana Francielle Marques[1], Heloá Robbi[1], Fabianne Altruda de Moraes Costa Carlesse[1,2], Adriana Seber[1,2]

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2. Instituto de Oncologia Pediátrica – GRAACC/UNIFESP – São Paulo/SP

INTRODUCTION:

Severe aplastic anemia (SAA) in pediatric patients presents a therapeutic challenge, especially when a matched sibling donor is unavailable and a haploidentical hematopoietic stem cell transplant (HCT) becomes necessary. Haploidentical transplantation using post-transplant cyclophosphamide (PT-Cy), while effective in reducing graft-versus-host disease, is associated with delayed immune reconstitution and increased susceptibility to opportunistic infections.

The objective of this case report is to describe the clinical course, complications, and outcome of a pediatric patient with SAA who developed secondary graft failure related to refractory cytomegalovirus (CMV) infection after haploidentical HCT.

RESULTS:

A 4-year-old previously healthy female was diagnosed with SAA after Influenza A infection. With no suitable matched donor available, she underwent haploidentical HSCT from her younger brother following judicial authorization. Conditioning included ATG, fludarabine, cyclophosphamide, and TBI. GvHD prophylaxis was with PT-Cy, MMF, and tacrolimus. Stem cell source was bone marrow (CD34+ 7.6x10^6/kg). Neutrophil engraftment occurred on day +20; no platelet recovery. Complete donor chimerism was observed on day +28. CMV prophylaxis with letermovir was started on day +5 (R+/D+). By day +18, CMV viremia rose and optic neuritis was diagnosed; ganciclovir was started, but CMV DNAemia progressed. Colon biopsies confirmed CMV colitis. Therapy was switched to foscarnet, but without virologic control. Ganciclovir was reintroduced while awaiting access to maribavir, which was delayed until day +58, after graft failure had occurred. She also developed invasive pulmonary aspergillosis. A second haploidentical HSCT using her father as donor was performed on day +77. One-day conditioning regimen included alemtuzumab, fludarabine, cyclophosphamide, and TBI. Peripheral blood was the stem cell source (CD34+10x106/kg); GvHD prophylaxis included tacrolimus and MMF. Neutrophil engraftment occurred on day +20 and platelet engraftment on day +52. CMV therapy with foscarnet and maribavir was continued until day +110. The patient later developed CMV-associated duodenal perforation, requiring another antiviral course. At day +337 post-second HSCT, she remains clinically stable with no new CMV or fungal complications.

CONCLUSION:

This case highlights the importance of early access to effective antiviral therapies in pediatric HCT. Delayed availability of maribavir—a low-toxicity option not yet approved for pediatric use in Brazil—contributed to prolonged CMV replication and graft loss. Regulatory approval of antiviral agents like maribavir and foscarnet for children is urgently needed to avoid avoidable complications in high-risk transplant settings.

KEYWORDS: Graft failure, cytomegalovirus, maribavir

SEVERE STAPHYLOCOCCAL INFECTION ASSOCIATED WITH THE USE OF IBRUTINIB TO TREAT CHRONIC GRAFT-VERSUS-HOST DISEASE

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INTRODUCTION:

Graft-versus-host disease (GVHD) is one of the leading causes of morbidity and mortality following hematopoietic stem cell transplantation (HCT), affecting 30–50% of the patients. Ibrutinib is an oral inhibitor of the Bruton's tyrosine kinase (BTK), which plays a crucial role in B-cell receptor signaling but also impacts the function of neutrophils and macrophages: inhibiting BTK, Ibrutinib impairs phagocytosis, inflammatory cytokine production and chemotaxis, leading to impaired neutrophil function and increased susceptibility to pyogenic bacterial infections. Reports of such infections remain scarce.

OBJECTIVE:

To report a case of severe staphylococcal infection associated with use of Ibrutinib in a pediatric patient with refractory chronic GVHD, highlighting a side effect that may not be rare among patients treated for this novel indication.

METHODS: Retrospective chart review.

RESULTS: The patient underwent an unrelated allogeneic HCT in May 2023

A male patient diagnosed with acute promyelocytic leukemia (APL) at the age of 16 months, underwent unrelated donor HCT in first morphological remission in May 2023 due to persistent molecular disease. Conditioning included BuCyMel, ATG, and GVHD prophylaxis, cyclosporine and methotrexate. The patient achieved complete donor chimerism and remission, but developed severe multisystemic chronic GVHD (skin, facia, oral mucosa, liver, and eyes), requiring multiple immunosuppressive therapies. Due to partial response to mesenchymal cells and ruxolitinib, with progression of cutaneous and hepatic GVHD, Ibrutinib was initiated in January, 2025. After three months on Ibrutinib, the patient developed purulent and crusted scalp lesions, with Staphylococcus aureus isolated from cultures. Treatment with oral linezolid for 21 days resulted in clinical resolution. The infectious process was attributed to BTK inhibition-induced neutrophil dysfunction, impairing phagocytosis and reactive oxygen species production.

CONCLUSION:

This case represents the first national report of severe staphylococcal infection associated with Ibrutinib use in a pediatric patient after HCT. It underscores the need for careful infectious monitoring and awareness of the underlying immunological mechanisms to ensure early recognition and safe use of emerging therapies such as Ibrutinib in the management of chronic/refractory GVHD.

THE ROLE OF THE CLINICAL NURSE SPECIALIST TO SET UP A COLLABORATIVE DATA AND BIOLOGICAL SAMPLE COLLECTION FOR A CROSS-SECTIONAL OBSERVATIONAL STUDY OF RENAL COMPLICATIONS IN PEDIATRIC HEMATOPOIETIC STEM CELL TRANSPLANTATION (HCT) SURVIVORS

Adriane da Silva Santos Ibanez[1], Ana Carolina Ribeiro Correa[1], Ana Cláudia Ramos Donatelli Bronzoni[1], Camilla Margarida Maria Parrode[1], Cintia Monteiro Lustosa[1], Cristiane Menezes Vitoria Alferi[1], Eduardo Freitas Hatanaka[3], Érica Almeida Viana[1], Erica Renata de Jesus[1], Fernanda de Oliveira Mota[1], Lahis Cardoso de Castro[1], Laís Lima Quintino[1,] Leticia Ribeiro Fargiorgio[1], Luciana dos Santos Domingues[1], Maria de Los Angeles Duran Salas[1], Maria Aparecida Aguiar da Silva[1], Maria Cristina Andrade[3], Maria Estela Shiroma[1], Maria Gabriela Alves Dias Matos[1], Maria Luiza Dautro Moreira do Val[3], Mayara Satsuki Kunii[1], Nathalia Gonçalves Rissardi[1], Olga Margareth Wanderley de Oliveira Felix[1], Raisa Machado Amaral[1], Paulo Cesar Koch Nogueira[3], Roseane Vasconcelos Gouveia[1,2], Valéria Cortez Ginani[1,2], Vanessa Aparecida do Nascimento Varjao[1], Vivian Antonia da Silva Soares[1], Yohanna Bastani de Mattos[1], Adriana Seber[1,2]

1- GRAACC/Unifesp.
2 – Samaritano - SP.
3 – Nefrologia Pediátrica do Departamento de Pediatria da UNIFESP – SP.

INTRODUCTION:

Several factors may be associated with renal dysfunction after HCT: underlying disease, conditioning regimen, the use of irradiation, nephrotoxic medications, infections, sinusoidal obstruction syndrome, transplant-associated thrombotic microangiopathy, graft-versus-host disease, and others. The survival of pediatric patients undergoing HCT has progressively increased. The objective of the proposed study was to evaluate the renal outcomes in pediatric patients after HCT.

The objective of this paper is to describe the Clinical Nurse Specialist role to develop a successful infrastructure for the research.

CASUISTIC:

All patients who underwent TCH ages between 0 to 18 years and who were being followed up at the TCH outpatient clinic between September 2024 and May 2025 were eligible for the collaborative study.

METHOD:

The pediatric nephrology group has partnered with the HCT team to study the renal outcomes of children after HCT as a cross-sectional observational study. The study was designed by both the nephrology team and the multidisciplinary TCH team, and aligned with the responsible laboratory. The study was approved

by the institutional Scientific Committee and by the University ethics committee. The nephologists partned with an external laboratory to run all research samples and infrastructure for picking up and transporting the samples was formally arranged. All data collection forms were defined. Our clinical specialist nurses had several meetings with the institutional leaders who would be involved in the process, educating the staff in these new procedures. Families consented the collection and the sequence of tubes to be collected was established, as well as the volume for each sample. It is important to emphasize that research samples are collected at the same time of the scheduled routine evaluations without any additional venipuncture. Results: The Clinical Nurse Specialist team is responsible for checking the scheduled post-HCT appointments, making the clinical pre-selection consultation, explaining the consent forms and scheduling the collection of the biological samples.

RESULTS:

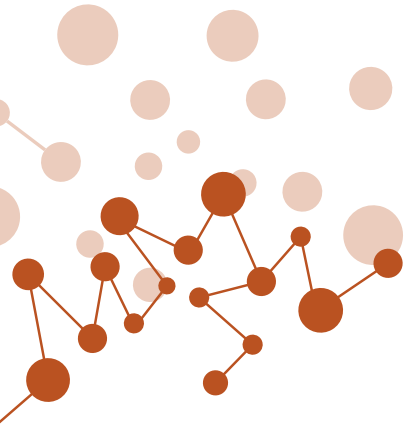
A total of 85 patients were already enrolled, 55% male, mean age 11 years(1-22). Time between HCT and study participation ranged from 1 month to 11 years post-HCT. No patients have refused enrollment until May 2025. Sample collections have been successful in all patients. Most of them had undergone Haploidentical HCT(48%), followed by HCT with unrelated donors(28%). Half of the patients had acute leukemias. Withing the next year we are expected to complete accrual and analyze the study results.

CONCLUSION:

The Clinical Nurse Specialist in Pediatric HCT has a central role in defining best strategies and achieving the completion of research protocols. These professionals have a central role in clinical HCT programs. The physician team, clinical nurses, multidisciplinary team, patients and families are extremely thankful for their participation.

KEY WORDS:

Hematopoietic Stem Cell Transplantation; Long Term Follow up; Onconephrology



INFECTIOUS COMPLICATIONS

ANTIFUNGAL PROPHYLAXIS IN A PRIVATE BONE MARROW TRANSPLANT CENTER: A ONE-YEAR EXPERIENCE

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INTRODUCTION:

Invasive fungal infections (IFIs) are common in hematopoietic stem cell transplant (HSCT) patients, especially during neutropenia. Prophylactic antifungal strategies with minimal drug exposure are under investigation to reduce toxicity and interactions.

OBJECTIVE:

To evaluate antifungal prophylaxis outcomes in a private hospital over one year of HSCT procedures.

METHODS:

Retrospective observational study with 17 patients, median age 47 (range 11–78): 11 allogeneic transplants (5 unrelated, 3 matched siblings, 3 haploidentical; 7 myeloablative, 4 reduced intensity for acute myeloid leukemia (4), acute lymphoblastic leukemia (2), sickle cell disease (2), myelofibrosis (2), myelodysplastic syndrome (1); and 6 autologous (3 for multiple myeloma, 3 for refractory lymphomas). Thirteen patients received fluconazole 300 mg/day PO, two were switched to IV micafungin 200 mg/day due to hepatotoxicity, and one received oral posaconazole 200 mg every 8 hours. Autologous recipients received prophylaxis only during aplasia until engraftment. Allogeneic recipients continued until neutrophil recovery and corticosteroid independence. All patients were housed in positive-pressure rooms with High efficiency particulate air (HEPA) filters and rigorous air-quality control.

RESULTS:

Median time to engraftment was 14.7 days (range 10–21). Autologous recipients used prophylaxis for <15 days; allogeneic patients used antifungal agents for a median of 31 days (range 22–90). No patients developed probable or confirmed IFIs. All patients were alive at analysis, with 0% transplant-related mortality and a median post-transplant survival of 240 days (range 25–457).

CONCLUSION:

Rational use of fluconazole during aplasia, combined with routine galactomannan testing (≥2 times/week) and effective protective environments, proved safe and effective. Most patients tolerated the strategy well, with minimal adverse effects.

KEYWORDS: Fungal, Transplant, fluconazole.

ASSESSMENT OF INACTIVATED VACCINE COVERAGES IN THE REVACCINATION PROGRAM OF HEMATOPOIETIC CELL TRANSPLANT RECIPIENTS AFTER THE SECOND YEAR OF TRANSPLANTATION

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INTRODUCTION:

After hematopoietic cell transplantation (HCT), patients lose immunity acquired through vaccination or lifelong infections and therefore need to be revaccinated. The program includes inactivated and attenuated vaccines and can be started from the third month of HCT, extending up to two years or more after transplantation when attenuated vaccines are started, if possible. Despite the availability of a free revaccination program for HCT recipients in Brazil, delays and failures in the revaccination schedule may occur. Few studies have evaluated vaccination coverage in long-term follow-up.

OBJECTIVES:

to evaluate the vaccination coverage that HSCT recipients received during post-HSCT follow-up.

METHODS:

We analyzed the vaccination records of outpatients who returned for scheduled visits and of patients who sent photos or copies of their vaccination cards after telephone contact. Three hundred and four (304) HCT recipients undergoing HCT from 1997 to 2019 were included. In our center, patients receive the referral letter for inactivated vaccines between d120 and d180. For each etiological agent, the doses received in both individualized and combined vaccines were considered. Annual vaccines, such as influenza and COVID-19, were not analyzed. Vaccination coverage was calculated by the number of patients receiving at least 1 dose of each vaccine out of the total number of patients analyzed.

RESULTS:

213 allogeneic and 91 autologous HCT recipients were included with a median age of 36 (1-73) years. Revaccination began at a median of 200 (77-3,486) days. The median follow-up was 2,192 (526-8,203) days. The best coverage was for the hepatitis B vaccine (98.7%) and the worst was for the HPV vaccine (6.9%). The table below shows the number of doses received and the coverage of each vaccine (Table 1). The coverage rates below 70% for the HAV, MCV, HPV, PCV and DTaP vaccines are probably due to the unavailability of these vaccines at the public vaccination centers in the year the patient was vaccinated. Despite the referral letter, we observed errors at the vaccination centers that could put this population at risk: 1) eight patients (2.6%) inadvertently received the oral polio vaccine, one of them without a previous record of inactivated polio vaccine (IPV); 2) number of doses lower or higher than prescribed; 3) failure to record which vaccine was administered (PCV or PPV).

CONCLUSION:

Coverage of inactivated vaccines was good (>78%) for half of the vaccines analyzed. Over time, an increasing number of vaccines has been included in the free vaccination program, which explains the low coverage of more recently introduced vaccines, such as HPV, acellular pertussis and pneumococcal conjugate. A better understanding of the reasons for vaccination delays or failures may help to overcome these problems. Periodic evaluation of vaccination cards is necessary to catch up the patients out of step with the revaccination calendar.

KEYWORDS: vaccine, inactivated vaccine, HSCT.

TABLE 01 - number of doses received and the coverage of each vaccine

Vaccine	Number of patients according to doses (%)				% Coverage
	None	1 dose	2 doses	≥ 3 doses	
Hepatitis B (HBV)	4 (1.3)	2 (0.6)	7 (2.3)	291 (95.7)	98.7
Tetanus, diphtheria (DT, dT)	7 (2.3)	4 (1.3)	12 (3.9)	281 (92.4)	97.7
Poliomyelitis (IPV)	18 (5.9)	7 (2.3)	17 (5.6)	262 (86.2)	94.1
Haemophylus influenza b (Hib)	29 (9.5)	19 (6.2)	42 (13.8)	214 (70.4)	90.4
Polysaccharide pneumococcal (PPV)	67 (22)	183 (60.2)	51 (16.7)	3 (1)	77.9
Hepatitis A (HAV)	112 (36.8)	32 (10.5)	157 (51.6)	3 (1)	63.1
Meningococcal conjugate (MCV)	127 (41.7)	48 (15.8)	119 (39.1)	10 (3.3)	58.2
Pneumococcal conjugate (PCV)	202 (66.4)	18 (5.9)	17 (5.6)	67 (22)	33.5
Acellular pertussis (Pa)	269 (88.5)	10 (3.3)	3 (1)	22 (7.2)	11.5
Human papillomavirus (HPV)	283 (93.1)	1 (0.3)	11 (3.6)	9 (2.9)	6.9

CYTOMEGALOVIRUS (CMV) REACTIVATION AS A SERIOUS INFECTIOUS COMPLICATION FOLLOWING ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Reactivation of latent Cytomegalovirus (CMV) infection is a common infectious complication following allogeneic hematopoietic stem cell transplantation and contributes to recipient morbidity and mortality. In addition, reactivation of the infection is associated with increased patient susceptibility to other conditions, such as Graft-versus-Host disease (GVHD) and bacterial or fungal infections, highlighting the need for continuous monitoring of patients and use of prophylaxis, such as Letermovir.

OBJECTIVES:

To elucidate the need of CMV prophylaxis based on the high chance of reactivation of latent infection following transplantation and its association with worse outcomes.

METHODS:

This study is a systematic review based on articles searched on the PubMed and Google Scholar platforms using the keywords "cytomegalovirus", "reactivation" and "hematopoietic stem cell transplantation". Six articles were included in the final analysis based on publishing date between 2020 and 2025, written in English and focused on CMV reactivation following allogeneic hematopoietic stem cell transplantation.

RESULTS:

Reactivation of CMV infection after hematopoietic cell transplantation is associated with increased morbidity and mortality of recipients, as well as reduced overall survival after transplantation. This event is related to the development of conditions such as pneumonia, encephalitis, gastroenteritis and hepatitis. In this sense, due to the state of myelosuppression to which the patient is subjected during the transplantation, reactivation of the virus becomes a common event, which entails the need for weekly monitoring.Considering the association of CMV infection with the increased risk of developing graft-versus-host disease and bacterial and fungal infections, prophylaxis with Letermovir is established with the aim of reducing post-transplant reactivation and preventing outcomes associated with decreased survival, since the time after transplantation associated with reactivation is approximately 35 days.

CONCLUSION:

Reactivation of latent CMV infection is an opportunistic infectious complication that requires intervention to reduce organ damage and other associated events, such as GVHD and other opportunistic infections. Therefore, prophylaxis with Letermovir is necessary to increase patient survival and reduce morbidity and mortality associated with CMV post-transplantation.

KEYWORDS:

Reactivation, Cytomegalovirus, hematopoietic stem cell transplantation

CYTOMEGALOVIRUS INFECTION AMONG BONE MARROW TRANSPLANT PATIENTS IN BRAZIL OVER THE LAST DECADE: A DESCRIPTIVE ANALYSIS

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INTRODUCTION

Cytomegalovirus (CMV) is one of the main opportunistic pathogens affecting patients undergoing bone marrow transplantation (BMT). Due to the need for immunosuppression during transplantation, patients become highly susceptible to opportunistic infections, often acquired within healthcare institutions during their post-transplant recovery.

This summary aims to provide a descriptive analysis of the sociodemographic and clinical characteristics of patients diagnosed with CMV following bone marrow transplantation.

METHODOLOGY

Public data were collected from the Hospitalization Information System (SIH) of the Brazilian Ministry of Health, focusing on secondary diagnoses of CMV (ICD-10 B25) among procedures such as allogeneic and autologous bone marrow transplants, follow-up of such transplants, and treatment of complications related to them, between 2015 and 2024. The dataset was cleaned and organized using R statistical software, and results were described using absolute (n) and relative (%) frequencies.

RESULTS

A total of 450 hospitalizations were recorded with CMV as a secondary diagnosis following BMT over the past decade. The sociodemographic profile showed a predominance of male patients (65.8%), while females accounted for 34.2%. Regarding age distribution, nearly half of the patients (48.2%) were aged 0–19 years, followed by 35.1% aged 20–49, and 16.4% aged 50–69. As for race/ethnicity, the majority self-identified as white (77.6%), with 21.8% identifying as Black, and 0.7% of records lacking information on this variable.

Clinically, 98.2% of patients survived hospitalization, while 1.8% died. Most hospital stays lasted between 11 and 20 days (58.0%), followed by 23.6% lasting more than 20 days and 18.4% lasting up to 10 days. Use of the ICU was rare, with only 1.1% of patients requiring it. The primary diagnoses leading to hospitalization for BMT included acute leukemias (50%), myelodysplastic syndromes (17.6%), and Hodgkin's disease (8.8%), among others.

CONCLUSION

The data reveal a predominantly young, male, and white patient profile. Most hospitalizations lasted between 11 and 20 days, with minimal ICU usage. Despite the low lethality, strict infection control measures for CMV and other pathogens remain essential to protect transplant recipients.

CYTOMEGALOVIRUS INVOLVEMENT OF THE CENTRAL NERVOUS SYSTEM AFTER AUTOLOGOUS TRANSPLANTATION FOR PRIMARY CENTRAL NERVOUS SYSTEM LYMPHOMA: A RARE COMPLICATION

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INTRODUCTION:

Cytomegalovirus (CMV) reactivation is a known complication following allogeneic bone marrow transplantation, in which antiviral prophylaxis has become standard practice. In contrast, CMV reactivation in patients who undergo autologous stem cell transplantation (ASCT) remains poorly characterized. Autograft recipients are considered to have low risk of CMV reactivation or end-organ disease due to transient immunodeficiency, although most patients received intensive chemotherapy, including Rituximab-based regimens and high-dose steroids prior to ASCT.

OBJECTIVES:

Determine which are the risk factors linked to CMV disease and assess the need for heightened clinical suspicion and the feasibility of earlier diagnostic testing in high-risk patients.

METHODS:

Retrospective review of medical records and literature.

RESULTS:

A 65-year-old male, diagnosed with primary CNS lymphoma in 2024, underwent R-Matrix protocol, followed by ASCT in May 2025. He had already received a cumulative steroid dose equivalent to 5.6g of prednisone and had a previously surgical wound infection with C. albicans and S. epidermidis. On day +5 post-ASCT, cefepime was initiated for neutropenic fever, escalated to meropenem after 3 days. He also had mild respiratory symptoms due to a Rhinovirus infection. After 7 days of antibiotic therapy, all blood cultures were negative. Neutrophil engraftment was achieved by day +10. However, the patient continued to experience daily isolated fever. The diagnostic workup included: sinus and chest computed tomography (CT); blood polymerase chain reaction (PCR) testing for Dengue virus, Mycobacterium tuberculosis, Epstein-Barr, Toxoplasma gondii and CMV; Aspergillus antigen testing on blood; bronchoalveolar lavage (due to a cavitated lesion on chest CT) with further testing for CMV, fungi and mycobacteria; and cerebrospinal fluid (CSF) analysis due to the onset of altered mental status, including confusion and drowsiness. CMV PCR in blood was positive (viral load: 9130UI/mL), and CSF analysis also exhibited CMV positivity. Given the evidence of CMV infection, ganciclovir was initiated. A CMV PCR in bronchoalveolar lavage is pending. All other investigations were negative. His immune reconstitution profile included immunoglobulins A, G and M of, respectively, 70.6, 282 and 8.3mg/dL, and CD4+ lymphocytes of 625/mm3. Due to the consideration of a grade 3 infection, prompt Intravenous Immunoglobulin replacement was initiated. Conclusion:This case highlights a

disseminated CMV (CNS and blood) after ASCT, likely triggered by intense immunosuppression followed by viral reactivation. Although not common in autologous recipients, the patient's persistent fever, confusional state and prior use of high-dose steroids and Rituximab, underscores the importance of heightened clinical suspicion in high-risk patients. Further research must prioritize rigorous risk stratification to validate the role of early molecular testing.

KEYWORDS: Cytomegalovius, Reactivation, Autologous stem cell transplantation.

DENGUE VIRUS TYPE 2 INFECTION IDENTIFIED BY METAGENOMIC ANALYSIS IN A HAPLOIDENTICAL HSCT RECIPIENT: A CASE REPORT

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INTRODUCTION:

Dengue is endemic in tropical countries such as Brazil. Although classical transmission occurs via mosquito vectors, transfusion-related cases—and more rarely, hematopoietic stem cell transplant (HSCT)-related cases—have been reported, particularly during outbreaks. In immunocompromised patients, dengue infection can be severe and prolonged. We report a case of dengue virus type 2 (DENV-2) infection in a haploidentical HSCT recipient with graft failure, raising the suspicion of non-vectorial transmission.

METHODS:

Case report based on clinical chart review and laboratory data, including plasma RNA metagenomic analysis.

CASE REPORT:

A 45-year-old male with FLT3-ITD+/NPM1+ AML underwent haploidentical allogeneic HSCT with a myeloablative conditioning regimen (FluBu4), receiving 5.565 x 10^6 CD34+ cells/kg. Post-transplant complications included febrile neutropenia, retinal hemorrhage, CMV reactivation, Clostridium difficile colitis, progressive hyperbilirubinemia with suspected acute graft-versus-host disease (GVHD), and acute kidney injury. From day +28, he experienced progressive chimerism loss, with complete donor cell loss by day +61. Due to persistent fever, pancytopenia, and graft failure despite broad-spectrum antibiotics, plasma metagenomic RNA sequencing was performed, revealing DENV-2 RNA. Additional tests ruled out other infectious etiologies, except for colonization with carbapenemase-producing Klebsiella pneumoniae and vancomycin-resistant Enterococcus in catheter cultures. Respiratory viral panel was negative. The patient developed multiorgan failure and severe functional decline (ECOG 4), leading to a family meeting and transition to palliative care.

CONCLUSION:

This case underscores the importance of considering dengue, particularly DENV-2, as a potential cause of graft failure in HSCT recipients, especially in endemic regions. Plasma RNA metagenomics proved crucial for differential diagnosis in a complex immunosuppressed context. The possibility of non-vectorial transmission—via transfusion or graft—highlights the need for stringent screening protocols and heightened surveillance during arbovirus outbreaks in transplant settings.

KEYWORDS: Dengue virus; Hematopoietic stem cell transplantation; Metagenomics

DISSEMINATED TUBERCULOSIS AFTER AXICABTAGENE CILOLEUCEL THERAPY IN A PATIENT WITH RELAPSED/REFRACTORY DIFFUSE LARGE B CELL LYMPHOMA: A CASE REPORT

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INTRODUCTION:

Disseminated tuberculosis is a severe form of Mycobacterium tuberculosis infection, characterized by the spread of bacilli to multiple organs and systems, and is associated with high morbidity and mortality. In patients undergoing chimeric antigen receptor (CAR) T-cell therapy, disseminated tuberculosis may present atypically and aggressively, requiring early diagnosis and prompt treatment.

OBJECTIVES:

To report an atypical and severe case of disseminated tuberculosis following CAR-T cell therapy, and to underscore the importance of timely recognition, accurate diagnosis, and appropriate management of this potentially life-threatening complication.

METHODS:

Retrospective review of medical records and literature.

RESULTS:

A 22-year-old male with relapsed/refractory Diffuse Large B Cell Lymphoma (R/R DLBCL) underwent car-t in July 2024, developing grade 1 cytokine release syndrome and immune effector cell-associated neurotoxicity syndrome, and was discharged on day +15 post-axicabtagene ciloleucel (axi-cel). By day +103, he presented to the emergency department with fever, headache, confusion, and seizures. Workup including brain magnetic resonance imaging and cerebrospinal fluid analysis was consistent with Mycobacterium tuberculosis, with no evidence of pulmonary infection on image by the time. On day +128, a new febrile episode occurred. Infectious screening identified pulmonary aspergillosis, evidenced by micronodules and positive galactomannan, prompting initiation of amphotericin B. Subsequent bronchoalveolar lavage confirmed tuberculosis, establishing a diagnosis of disseminated infection. By then, the cumulative steroid dose was equivalent to 5 g of prednisone. Despite RIPE therapy and infection control, the patient died on day +153 post-CART due to a hemorrhagic complication.

CONCLUSION:

CAR-T cell therapy represents a major advancement in the treatment of R/R DLBCL but is associated with immune-mediated toxicities such as B-cell aplasia and hypogammaglobulinemia, together with the use of high-dose steroid therapy for its immune-related adverse events, increasing infection risk. To date, tuberculosis has been rarely reported in CAR-T recipients, highlighting the need for further studies and consensus on its prophylaxis, particularly in endemic regions.

KEYWORDS: CAR-T cell therapy, Tuberculosis, Lymphoma.

DOES THE NEW DEFINITION OF REFRACTORY CMV IMPROVE CLINICAL RELEVANCE? INSIGHTS FROM A SINGLE-CENTER COHORT

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BACKGROUND:

Cytomegalovirus (CMV) remains a significant cause of morbidity and mortality among recipients of allogeneic hematopoietic cell transplantation (allo-HCT). In Brazil, where CMV seroprevalence exceeds 90%, reactivation is frequent and poses a major clinical challenge. In 2024, a revised definition of refractory CMV (refCMV) was introduced to enhance the standardization of clinical trials and enable earlier identification of suboptimal treatment response. However, the impact of this new definition in clinical settings remains unclear.

OBJECTIVE:

To assess the effect of the updated refCMV definition on its frequency and to evaluate whether classification differences are associated with clinical outcomes.

Methods: We conducted a retrospective observational cohort study including all patients who underwent allo-HCT at a single center between 2016 and 2023. CMV reactivation episodes were identified and classified using both the previous and the 2024 consensus definitions. The previous definition categorized refCMV as either: (i) proven, defined by an increase in viral load after ≥2 weeks of appropriate therapy, or (ii) probable, defined by <1 log10 decline in viral load. The updated 2024 definition considers refCMV as CMV viremia that increases by >1 log10 or persists (≤1 log10 change) after ≥14 days of appropriate antiviral treatment. We excluded episodes with treatment less than 14 days.

RESULTS:

Among 260 Allo-HCT recipients, 93% were CMV-seropositive, and 42% received haploidentical transplants. CMV clinically significant reactivation occurred in 146 patients (41%), totaling 149 episodes. According to the previous definition, 68 (46%) and 7 (5%) episodes were classified as probable and proven refCMV, respectively. Using the updated definition, 81 episodes (54%) met criteria for refCMV. Notably, 54% and 72% of cases in the probable and proven groups, respectively, had viral loads >1,000 IU/mL, while 51% of those classified under the updated definition had viral loads <1,000 IU/mL. CMV disease was documented in 10%, 57%, and 12% of cases in the probable, proven, and updated refCMV groups, respectively (p=0.002). Corresponding mortality rates were 26%, 86%, and 27% (p=0.01).

CONCLUSIONS:

The updated 2024 refractory CMV (refCMV) definition resulted in a modest yet clinically meaningful increase in refractory case classifications. Importantly, this revised classification demonstrated stronger correlation with key clinical outcomes - including CMV disease incidence and reduced mortality - compared to the traditional 'proven refractory' category. The new criteria better reflect real-world clinical practice and facilitate earlier identification of patients who would benefit from therapeutic modification.

EARLY ORAL DETECTION OF CMV WITHOUT VIREMIA MODIFYING CLINICAL MANAGEMENT IN A PATIENT WITH GVHD AFTER ALLO-HCT: IMPORTANCE OF DIFFERENTIAL DIAGNOSIS

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INTRODUCTION:

Graft-versus-host disease (GVHD) and opportunistic infections are common complications in patients undergoing allogeneic hematopoietic cell transplantation (allo-HCT), significantly impacting morbidity and mortality when not promptly recognized and managed. HCT recipients who are CMV-seropositive and receive grafts from CMV IgG-negative donors are at high risk of developing clinically significant CMV infections during immunosuppression. These infections can manifest as pneumonia, retinitis, bone marrow suppression, colitis, hepatitis, and encephalitis. Therefore, early and accurate diagnosis of CMV reactivation is crucial for reducing complications and improving outcomes. Plasma CMV DNA monitoring is the current gold standard for early detection and treatment.

OBJECTIVE:

To report a case of oral CMV lesions without detectable plasma viremia, occurring in the context of overlapping GVHD.

METHODS: Case report.

RESULTS:

A 3-year-old female diagnosed with osteopetrosis due to a TCIRG1 mutation underwent allo-HCT from an unrelated HLA-identical donor. The donor was CMV IgG-negative, while the recipient was CMV IgG-positive. Peripheral blood stem cells were used as the graft source. Conditioning consisted of fludarabine, busulfan, and cyclophosphamide (FLUBUCy), and GVHD prophylaxis included cyclosporine and mycophenolate. The patient exhibited delayed engraftment (day +35), and chimerism analysis on day +100 confirmed full donor chimerism. On day +55, she developed acute skin GVHD overlapping with chronic involvement (skin, oral mucosa, and gastrointestinal tract), treated with methylprednisolone, cyclosporine A, immunoglobulin, and ruxolitinib. CMV reactivation was detected on day +25 and treated with ganciclovir, which was discontinued following clinical and virological improvement. Reactivation occurred again on day +87, prompting reinitiation of antiviral therapy. On day +166, plasma CMV was undetectable, and antiviral therapy was stopped. On day +194, the patient was rehospitalized due to worsening oral lesions, diarrhea, anorexia, dehydration, and extensive ulcerative lesions in the

oral cavity. Despite negative plasma CMV, oral swab PCR and cytology were positive for CMV on day +203. Ganciclovir was resumed, resulting in marked clinical improvement within 48 hours. Plasma CMV became positive three days after the oral swab. Oral biopsies confirmed chronic GVHD with reactive mucositis and were positive for CMV, while other viral pathogens were excluded (Table1). As of day +227, the patient remains plasma CMV-negative, with improved oral lesions and GVHD symptoms, and antiviral therapy has been discontinued (Figure1).

CONCLUSION:

This case highlights the importance of differential diagnosis in GVHD patients and underscores the potential for localized CMV infection with negative plasma viremia. Oral CMV disease should be considered in symptomatic patients, even in the absence of systemic viral detection.

KEYWORDS: Cytomegalovirus (CMV), Graft-versus-Host Disease (GVHD), Allogeneic Hematopoietic Cell Transplantation (allo-HCT), Oral Lesions

D+	PCR Quantitative Result	LOG
D+5	0	0,00
D+17	0	0,00
D+25	142	2,15
D+28	292	2,46
D+31	674	2,82
D+34	179	2,25
D+39	0	0,00
D+45	0	0,00
D+53	0	0,00
D+80	0	0,00
D+87	298	2,47
D+94	496	2,69
D+101	0	0,00
D+104	117	2,06
D+109	132	2,12
D+117	0	0,00
D+143	312	2,49
D+146	279	2,43
D+155	0	0,00
D+166	0	0,00
D+194	0	0,00
D+203	0	0,00
D+206	234	2,36
D+208	240	2,38
D+213	472	2,67
D+216	0	0,00
D+220	204	2,31
D+223	0	0,00
D+227	0	0,00
D+231	0	0,00
D+234	0	0,00

FIGURE1: Longitudinal monitoring of CMV PCR quantification and Ganciclovir Therapy in a pediatric post-HCT pacient with Osteopetrosis

Table 1: Exam Panel

PCR	D+203		D+206	
	Mouth	Blood	Mouth	Blood
CMV	+	-	+	-
Zoster	-	-	-	-
HHV6	-	-	-	-
EBV	-	-	-	-
HHV1	-	-	-	-
HHV2	-	-	-	-
Dengue	NA	-	NA	-
Zikavirus	NA	-	-	-
Chikungunha	NA	-	-	-
Mycoplasma	-	NA	-	NA

ENDEMIC INFECTIONS FOLLOWING CAR19-T-CELL THERAPY: REAL-LIFE EVIDENCE

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INTRODUCTION:

Chimeric antigen receptor T-cell (CAR-T) therapy is a promising treatment for refractory hematologic malignancies, but patients may experience prolonged cytopenia and compromised immune function post-treatment. The lymphodepletion chemotherapy administered before CAR-T infusion also induces cytopenia and weakens mucosal barriers. Despite epidemiological studies, most data come from small cohorts and clinical trials, with limited understanding of infection patterns in patients from endemic areas. In low-income countries, delays in CAR-T infusion often require prolonged bridge therapies, which increases the risk of immunosuppression, as well as the development of ICANS and CRS.

OBJECTIVE:

This study aims to describe the clinical and demographic characteristics of patients undergoing CAR-T therapy at a Brazilian Cancer Center, identify infectious complications post-lymphodepletion and CAR-T infusion, and explore the role of endemic infections in Brazil.

RESULTS:

This study included 23 consecutive patients with hematological malignancies who underwent CAR-T cell therapy. Among them, 18 were Diffuse Large B-cell Lymphoma (DLBCL), two were Follicular Lymphoma (FL) and three Acute Lymphoblastic Leukemia (ALL). The median age was 54 years old, and most patients were male 15 (65.5%). There were 33 documented infections in 14 of 23 patients (60.8%) with an infection density of 1.43 infections for every 100 days at risk. Eighteen infections (9 bacterial, 4 viral, 4 fungal and 1 protozoan) occurred in 10 patients within the first 30 days of CAR-T infusion, and fifteen infections (4 bacterial, 9 viral, 2 fungal), in 9 patients, after the first 30 days that followed infusion. Consistent with existing literature, we found the highest incidence of infections within the first month after CAR-T therapy, with bacterial infections, Candida, and CMV reactivation predominating during this period. Respiratory viral infections were more common after the first month. One patient presented with a late infection of disseminated tuberculosis, confirmed in the cerebrospinal fluid (CSF) and pulmonary disease as well.

CONCLUSION: The incidence of infections following CAR-T therapy is influenced by regional and local factors. Screening for endemic infectious diseases is crucial for guiding prophylactic measures and ensuring timely intervention.

KEYWORDS: CAR-T-Cell Therapy; Endemic infection, Immunocompromised host

EVALUATION OF A SCREENING PROTOCOL FOR SARS-COV-2 INFECTION IN HEMATOPOIETIC STEM CELL TRANSPLANT RECIPIENTS: A FOUR-YEAR RETROSPECTIVE ANALYSIS IN A PUBLIC HOSPITAL

Ivelise Giarolla[1], Ana Carolina Goulardin de Almeida[1], Roselene Mesquita Augusto Passos[1], Diego Cassola Pronunciato[1], Ana Paula de Castro Guima[1], Deyvid Fernando Mattei da Silva[1], Marco Antonio de Souza Brito[1]

1. Hospital de Transplantes Euryclides de Jesus Zerbini, São Paulo- SP

INTRODUCTION:

Patients undergoing hematopoietic stem cell transplantation (HSCT) are at increased risk for viral infections, including COVID-19, due to prolonged immunosuppression. In the context of the SARS-CoV-2 pandemic, the implementation of screening strategies has become essential to ensure the safety of this vulnerable population.

OBJECTIVE:

To evaluate the effectiveness of a screening protocol for SARS-CoV-2 infection, consisting of clinical triage and serological testing, applied to pre-HSCT patients from 2020 to 2024 in a tertiary public hospital.

METHODOLOGY:

During the pre-transplant period, specific recommendations were adopted for hematopoietic stem cell transplant (HSCT) candidates and donors to prevent SARS-CoV-2 infection. For transplant candidates: Home isolation was recommended for 14 days prior to the start of conditioning, along with mandatory SARS-CoV-2 testing with a negative result. In the case of a positive test, the transplant would be postponed for at least 30 days, requiring the absence of symptoms and two negative RT-PCR tests at least 15 days apart. Transplant-related procedures would be suspended for 14 to 21 days following close contact with an infected individual and would only resume after a negative RT-PCR test. For donors: Mandatory SARS-CoV-2 testing with a negative result was required. In the 28 days prior to donation, donors were advised to follow strict hygiene practices and avoid large gatherings.

RESULT:

Between 2020 and 2024, 579 tests for respiratory virus detection were analyzed. The majority of patients were male (58%), with a predominant age group between 60 and 69 (34%). Among the tests performed, 88% were negative for viral infections. SARS-CoV-2 (COVID-19) positivity was 6% (n = 33), with a predominance of asymptomatic cases (79%). Other detected viruses included rhinovirus (3%), influenza (1%), adenovirus (1%), and respiratory syncytial virus (RSV) (1%). Among the cases positive for non-COVID-19 respiratory viruses (n = 34), 91% were asymptomatic. All patients and donors diagnosed with COVID-19 followed the institutional protocol, which involved suspending cell collection and rescheduling the procedure after 30 days, provided they were asymptomatic and had negative serological tests. There was a single case of death due to COVID-19, occurring on day +12 following autologous transplantation. The patient had previously tested positive for COVID-19, was asymptomatic, and had a negative RT-PCR after 30 days, at which point the transplant was performed.

CONCLUSION:

Most SARS-CoV-2-positive cases were asymptomatic, highlighting the importance of screening even without symptoms. The strict implementation of the institutional protocol ensured the safety of procedures, and the single recorded death underscores the need for ongoing vigilance.

KEYWORDS: Hematopoietic Stem Cell Transplantation (HSCT), Pre-transplant Screening, COVID-19

FANNING THE FLAMES: HEMATOPOIETIC STEM CELL TRANSPLANT COMPLICATED BY TRANSFUSION-TRANSMITTED DENGUE

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INTRODUCTION:

Though rare, fatal dengue transmission through blood products has been reported in Hematopoietic Stem Cell Transplant (HSCT) recipients, posing a concern in dengue-endemic regions.

OBJECTIVE:

To describe a case of transfusion-transmitted dengue in an autologous HSCT patient.

METHODS:

Case report with patient consent.

RESULTS:

A 70-year-old woman with IgG kappa multiple myeloma, in very good partial response after 4 cycles of VRd, underwent autologous HSCT with melphalan 200 mg/m^2 conditioning and infusion of 2.68 × 10^6 CD34+ cells/kg. On day +5, she developed severe epistaxis and thrombocytopenia (26,000/mm^3) and received apheresis platelet transfusion (filtered, irradiated). Neutrophil engraftment occurred on day +10, platelet engraftment on day +11. On day +12, she developed postural hypotension and syncope, with progressive liver enzyme elevation. Amid a local dengue outbreak, NS1 antigen was positive, confirmed by PCR. Aggressive IV hydration and supportive care led to clinical recovery. She was discharged on day +24. Investigation revealed the transfused platelet unit tested positive for dengue virus (DENV-1) by PCR. Both the patient's and donor's samples matched. The donor later reported dengue-like symptoms but did not seek care or notify the blood center. All remaining blood products from this donor were discarded.

CONCLUSION:

In dengue-endemic areas or outbreaks, dengue detection and pathogen inactivation measures should be considered in HSCT settings. A high index of clinical suspicion is crucial for timely diagnosis and intervention.

KEYWORDS:

Dengue, Transfusion, Transplant.

FATAL MAGNUSOMYCES CAPITATUM SEPSIS FOLLOWING ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANT: AN ECHINOCANDIN BREAKTHROUGH INFECTION IN A RECIPIENT PREVIOUSLY EXPOSED TO ECULIZUMAB.

João Antônio Gonçalves Garreta Prats[1], Phillip Scheinberg[1], Anna Carolinne Leal do Nascimento[1], Aline Gimenez Guerra[2], Lis Moreno de Oliveira Kalid[3], Paola Cappellano[4], Jessica Fernandes Ramos[5]

1. *Hospital A Beneficência Portuguesa de São Paulo*
2. *Hospital Samaritano de São Paulo*
3. *Hospital Regional de Juazeiro*
4. *Grupo Fleury*
5. *Hospital Sírio-Libanês*

INTRODUCTION:

Data on allogeneic HSCT in PNH are limited, and in the most extensive series published, the leading cause of death was infection. While the current use of complement inhibitors is strongly associated with increased infections due to capsulated germs, limited data on fungal infections make it challenging to establish strategies for prophylaxis in this context.

OBJECTIVE: To report a rare agent causing fungemia and death after allo HSCT in a patient using eculizumab.

CASE REPORT:

A 64-year-old male patient with high-risk MDS with GATA-2 mutation secondary to PNH, on continuous use of biweekly eculizumab in the last two years, was admitted with stable disease after six cycles of azacitidine and venetoclax. He underwent haploidentical HSCT with major ABO incompatibility with FluMelTBI200 conditioning and graft-versus-host disease (GVHD) prophylaxis with post-cyclophosphamide, mycophenolate, and tacrolimus. He continued the use of eculizumab in the peri-transplant period and received acyclovir and anidulafungin prophylaxis during neutropenia. He presented the first febrile neutropenia on D+2 with documented bloodstream infection by Pseudomonas aeruginosa and ESBL E. coli. He received cefepime, which was later escalated to meropenem and finally de-escalated to levofloxacin based on the susceptibility test. On D+5, the patient developed colitis due to Clostridioides difficile and started treatment with oral vancomycin. The patient had a good clinical evolution, was afebrile, and had improved diarrhea until D+9 when he presented a new NF with septic shock. Despite a broad antimicrobial regimen with meropenem, amikacin, teicoplanin, and partial negative cultures, the patient died on D+10. Approximately 48 hours after death, filamentous fungus growth was observed in blood cultures. Mass spectrometry (MALDI-TOF) identified the fungus as Magnusomyces capitatus (formerly Geotrichum capitatum).

DISCUSSION:

Magnusomyces capitatus is a dimorphic fungus found in the environment and as part of the skin, digestive tract, and/or respiratory tract microbiota. It is a rare mold with few case reports related to solid organ transplant and neutropenia. Patients using complement inhibitors, as in our case, are not classified as at high risk for invasive fungal infections. Microbiology data shows higher in vitro activity for azoles, while echinocandins are not an option for these infections. The diagnosis of these agents has improved in recent years with the dissemination of the MALDI-TOF method. Further studies are needed to establish strategies for preventing and treating infectious complications specific to patients receiving complement inhibitors in the peri transplant period, and additional caution in patients receiving equinocandin as prophylaxis may be needed.

FULMINANT COLITIS DUE TO CLOSTRIDIOIDES DIFFICILE FOLLOWING HAPLOIDENTICAL TRANSPLANT IN A PATIENT WITH PHILADELPHIA-POSITIVE MIXED PHENOTYPE ACUTE LEUKEMIA

Autores: Talita Carvalho Siqueira[1], Nyara Walleska Maciel de França[1], Carla Campos Miranda[1], Ana Paula de Azambuja[1], Rafael Marchesini[1], Felipe Borges Patrício[1], Mathias Regis Modesto[1], Ricardo Romano[1], Guilherme Cecchetti[1], Silvio Henrique Vieira Freitas[1], Marcos Roberto Curcio Pereira[1], Gabriela UgioniDuarte[1], Guilherme Lourenço[1], Ediely Layana Oliveira Coletto[1], Samir Nabhan [1], Daniela Setubal[1], Caroline Bonamin Sola [1], Glaucia Tagliari[1], Miguel Pedro de Queiroz Neto[1], Giovani Breda[1], Vaneuza Araújo Moreira Funke[1]

1. Bone Marrow Transplant Center of the Clinical Hospital Complex of the Federal University of Paraná (CHC-UFPR)

INTRODUCTION

Fulminant colitis is a rare and potentially fatal complication of Clostridioides difficile infection, especially in immunocompromised individuals such as recipients of hematopoietic stem cell transplantation (HSCT). Its clinical course is often abrupt and severe, requiring early diagnosis and intervention.

OBJECTIVE

To report a case of fulminant colitis due to C. difficile following haploidentical HSCT in a patient with Philadelphia-positive mixed phenotype acute leukemia (MPAL), highlighting diagnostic and therapeutic challenges.

METHODS

This is a descriptive case report based on clinical, laboratory, imaging, and histopathological data, including review of complementary exams and post-HSCT clinical evolution. A literature review was conducted in PUBMED, MEDLINE, and Science Direct databases regarding C. difficile complications in HSCT recipients.

RESULTS

A 30-year-old female patient was diagnosed with B/myeloid MPAL and underwent haploidentical HSCT. Immunophenotyping showed 43.7% B-blasts (CD79a+, CD19+, CD10+, CD20+, CD22+), with aberrant expression of myeloperoxidase, myeloid markers (CD13, CD33, CD66c), CD304, and CD73. She presented liver infiltration, complex karyotype, and the BCR::ABL1 p190 transcript.

She was treated with Hyper-CVAD and imatinib, achieving measurable residual disease (MRD) of 0.06%. Conditioning included fludarabine and total body irradiation. Discharged on day +18, she was readmitted on day +34 with hemorrhagic cystitis due to BK virus and adenovirus.

During hospitalization, she developed abdominal pain, distension, and vomiting, without diarrhea. Abdominal CT showed diffuse colonic wall thickening, mucosal enhancement, pericolonic fat stranding, free fluid, and pneumoperitoneum. On day +84, she progressed to septic shock and underwent total colectomy with ileostomy.

Histopathological analysis revealed extensive mucosal necrosis, epithelial sloughing, dense neutrophilic infiltrate, crypt abscesses, deep ulcerations, and transmural ischemia. These findings were consistent with extensive pseudomembranous colitis. She recovered well postoperatively and remains stable on outpatient follow-up.

CONCLUSION

This case illustrates an atypical and severe presentation of C. difficile colitis post-HSCT, without diarrhea and with negative toxin assay. Radiological and histopathological findings were key to diagnosis. Early clinical suspicion, comprehensive evaluation, and timely surgical management were essential for a favorable outcome.

Even in the absence of classical symptoms, C. difficile should be considered in the differential diagnosis of abdominal complications in immunocompromised patients.

KEYWORDS: Clostridioides difficile; fulminant colitis; hematopoietic stem cell transplantation; abdominal imaging

HERPES VIRUS 7- HHV7 INFECTIONS MAY MIMIC ACUTE GRAFT-VERSUS-HOST (GVHD) DISEASE AFTER PEDIATRIC HEMATOPOIETIC STEM CELL (HCT) TRANSPLANTS

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1. GRAACC, Sao Paulo - SP - Brasil.

INTRODUCTION:

The human herpesvirus (HHV) family are large DNA viruses that may present with latent infection and periodic reactivations. The primary HHV-7 infections occur in early childhood and it is one of the causes of febrile maculopapular rashes. In addition, HHV-7 has been found in other inflammatory skin diseases, such as psoriasis. It has lifelong cellular latency, being easily reactivated in the setting of immunodeficiency. However, the underlying pathological mechanism is still uncertain. Although clinically symptomatic infections are less common, 75% of children are HHV7-positive by 5 years of age.

OBJECTIVES:

To study the clinical presentation of HHV-7 infections in pediatric patients undergoing HCT. Methods: We included all children between 1 and 19 years of age after HCT who had a positive HHV-7 PCR between January 2023 and April 2025. Of these patients, 15 underwent myeloablative conditioning, 1 with ATG and 14 without, and 4 RIC, 1 with ATG and 3 with ATG as prophylaxis for GVHD. Regarding prophylaxis for GVHD, 2 used ATG-CNI-MTX, 1 ATG-PT Cy-CNI-MMF and 1 PT Cy-CNI-MMF in the RIC regimen. In the myeloablative regimen, 3 underwent CNI, 3 CNI-MTX and 10 PT Cy-CNI-MMF.All patients have weekly blood panherpes PCR performed weekly until D+100. It is also performed in all tissue samples obtained for differential diagnosis between GVHD and viral infections. Data were collected from the medical records.

RESULTS:

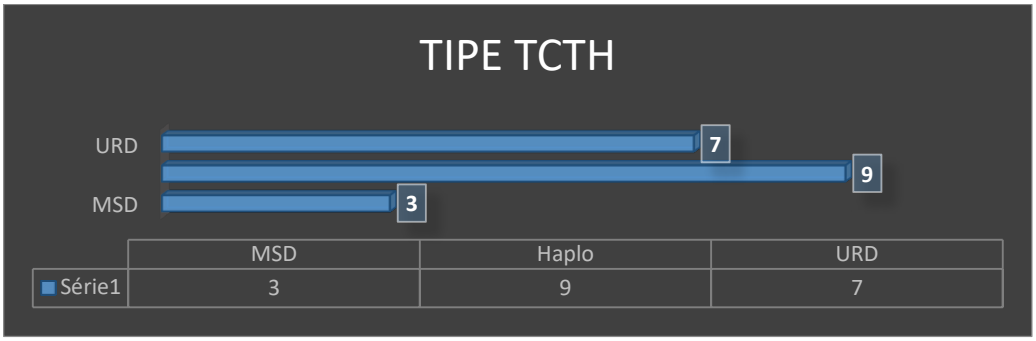
Of a total of 19 patients had at least one positive PCR for HHV-7, 9 females. Donors were matched sibling donors (MSD-3), haploidentical (Haplo-9) and unrelated donors (URD-7), with a prevalence of 3/20 MSD, 9/59 Haplo and 7/47 URD within the same period. The HHV-7 was detected after a median of 154 days after HCT (range 34 - 1387 days). 14/19 were still on immunosuppression at the time of reactivation, 5 of them being treat for acute and 9 for chronic GVHD. The HHV-7 was the only virus isolated in 8 patients and 9 had other simultaneous viral infections by the herpes family (1 CMV, 1

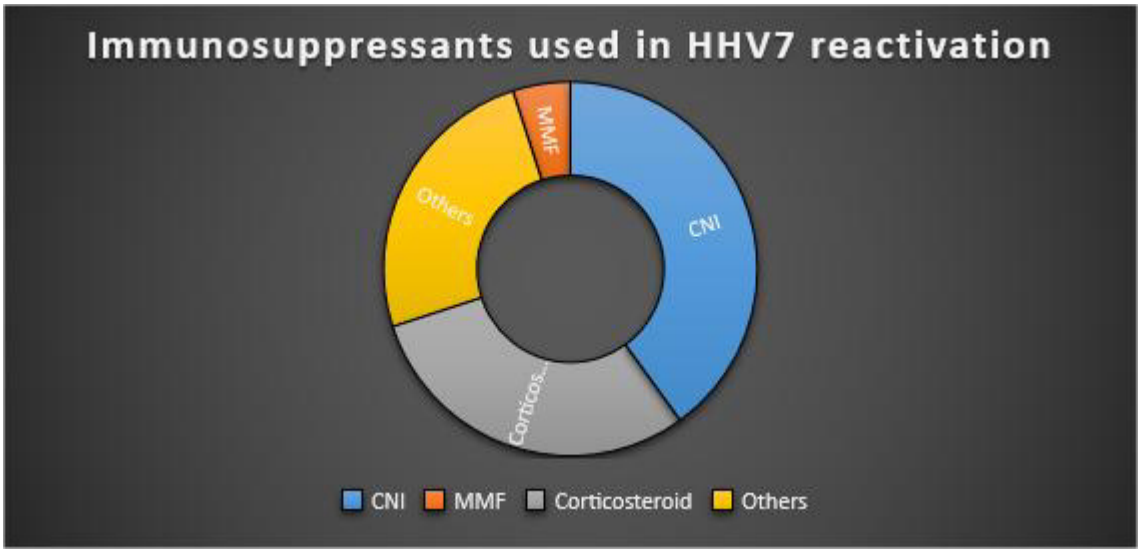
HSV1, 3 HHV6 and 4 EBV), Metapneumovirus and Adenovirus, one patient each. Only 3/19 patients were asymptomatic and the HHV-7 was detected on the weekly surveillance. Most infections (15) were identified in the gastrointestinal tract, mimicking GVHD; 4 were in the blood and 1 was in the bronchoalveolar lavage. Simultaneous GVHD and HHV7 were demonstrated in 5 patients. 3 had simultaneous HHV6 and HHV7. Symptoms were weight loss, anorexia and diarrhea. 17/19 patients were treated with ganciclovir or valganciclovir and had complete resolution of the symptoms.

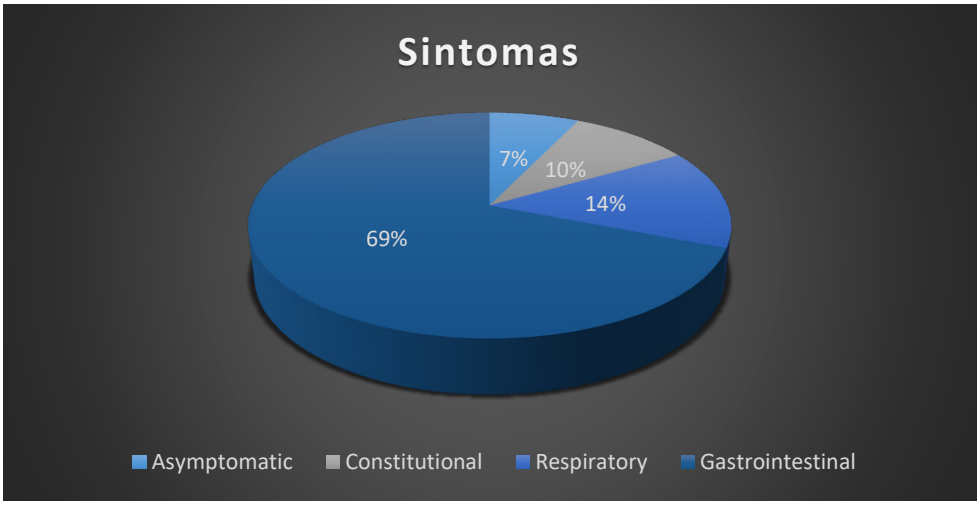
CONCLUSIONS:

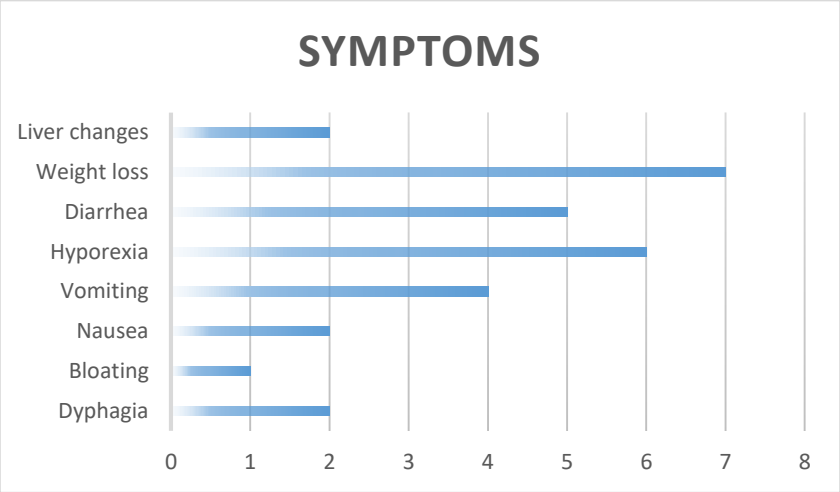
The vast majority of symptomatic HHV7 infections were in the GIT and all of them were treated in a timely manner, without complications. Joint infection with some virus of the herpes family was common, but with HHV6 they were lower than expected, being only in 3 cases. Early evaluation with endoscopy helped a lot to improve symptoms and early treatment.

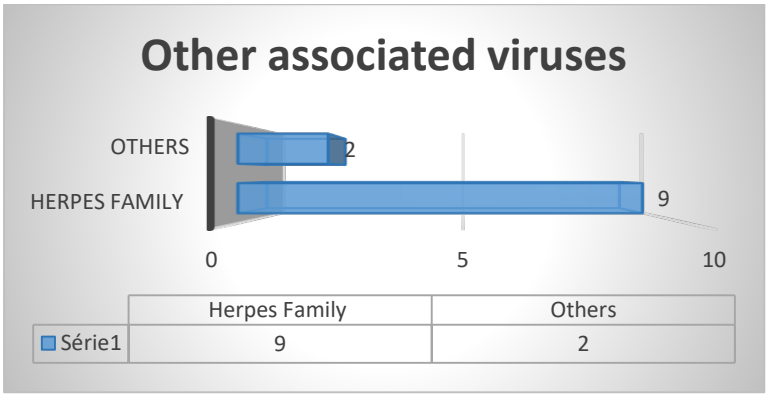
KEYWORDS: Hematopoietic stem cell; HHV7 infections, symtoms HHV7

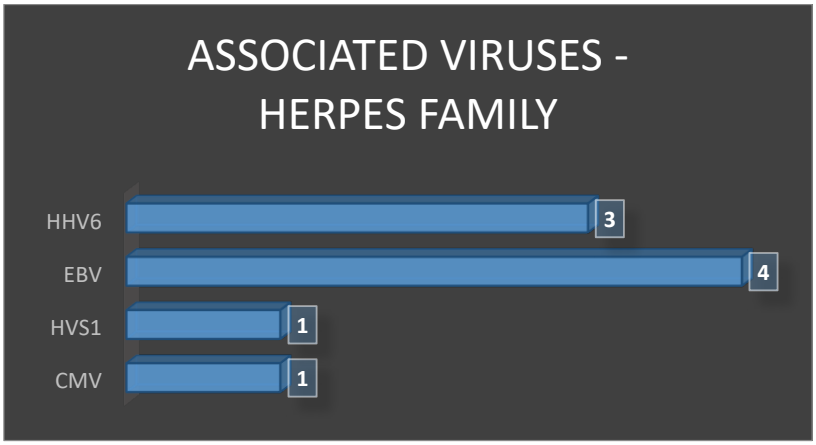

TIPE TCTH
URD
MSD
7
9
3
MSD
Haplo
URD
Série1
3
9
7


Immunosuppressants used in HHV7 reactivation
MMF
CNI
Others
Corticos...
CNI
MMF
Corticosteroid
Others


Sintomas
7%
10%
14%
69%
Asymptomatic
Constitutional
Respiratory
Gastrointestinal


SYMPTOMS
Liver changes
Weight loss
Diarrhea
Hyporexia
Vomiting
Nausea
Bloating
Dyphagia
0
1
2
3
4
5
6
7
8


Other associated viruses
OTHERS
HERPES FAMILY
2
9
0
5
10
Herpes Family
Others
Série1
9
2


ASSOCIATED VIRUSES - HERPES FAMILY
HHV6
EBV
HVS1
CMV
3
4
1
1

IMPACT OF EARLY CLINICALLY SIGNIFICANT CMV INFECTION IN HAPLOIDENTICAL HCT NOT RECEIVING LETERMOVIR PROPHYLAXIS

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2. Hospital Amaral Carvalho, Jaú;

INTRODUCTION:

In Brazil, the seroprevalence of CMV infection in hematopoietic cell transplant (HCT) recipients is over 90%, and the cumulative incidence (CI) of CMV reactivation in allogeneic HCT is around 70%. In this setting, the best strategy for CMV control is letermovir prophylaxis. However, pre-emptive ganciclovir is still largely used because foscarnet and letermovir are not available in public HCT centers in Brazil due to economic reasons. With the increasing use of PTCY beyond the scenario of haploidentical HCT, an increase in the frequency as well as an earlier occurrence of CMV reactivations have been observed.

OBJECTIVES:

This study aimed to evaluated the impact of early clinically significant CMV infection (early CMV-CS) in haploidentical HCT recipients.

METHODS:

Eighty-nine patients undergoing haploidentical HCT at the HCT Program of Hospital Amaral Carvalho were included from Oct 2021 to Dec 2023. Early CMV-CS was defined as CMV reactivation demanding ganciclovir (GCV) therapy and occurring prior to, or up to 2 days after neutrophil engraftment. CMV and HHV-6 monitoring was performed with quantitative PCR. Preemptive GCV was introduced according to a locally defined viral load (VL) cut-off. Treatment of HHV-6 with GCV was done in case of encephalitis or at physician discretion. The cumulative incidences of engraftment failure, bacterial infections, relapse, and overall survival were compared in patients with or without early CMV-CS.

RESULTS:

The CI of CMV and HHV-6 reactivation were 85% and 80%, respectively, and occurred at a median of 26 (3 to 164) days and 24 (3-94) days, respectively (Figure 1). Nineteen patients had early CMV reactivation, of which 15 (16.8%) received GCV preemptive therapy (early CMV-CS). Of the four untreated patients, three had low VL that turned negative spontaneously, and one patient died on the day of CMV reactivation. Early CMV-CS occurred at a median of 17 (3 to 21) days. Eight patients (53.3%) had reactivation before engraftment and seven (46.6%) on or after engraftment. Patients with early CMV-CS had a significantly higher CI of graft failure (13.3% vs 1.4%, p=0.02) and bacterial infections (93% vs 65%, p=0.021) than patients without early CMV-CS (Figure 2). No difference was observed in overall survival (50.8% vs 72.5%, p=0.17). Early HHV-6 reactivation had no impact on graft failure (0% vs 4.3%, p=0.34), bacterial infections (60% vs 73%, p=0.23) or overall survival (75% vs 67.4%, p=0.60) (Figure 3).

CONCLUSION:

In this study, approximately 17% of CMV-CS occurred before or within two days of engraftment and increased the risk of graft failure and bacterial infections probably due to ganciclovir myelotoxicity. Early HHV-6 reactivation did not affect neutrophil engraftment in this series. By reducing the probability of CMV reactivation, letermovir prophylaxis may prevent the occurrence of early CMV-CS and the undesirable GCV myelotoxicity at the time of engraftment.

KEYWORDS: CMV infection, haploidentical HCT , letermovir.

FIGURE 1. Cumulative incidence of CMV-CS in haplo HCT

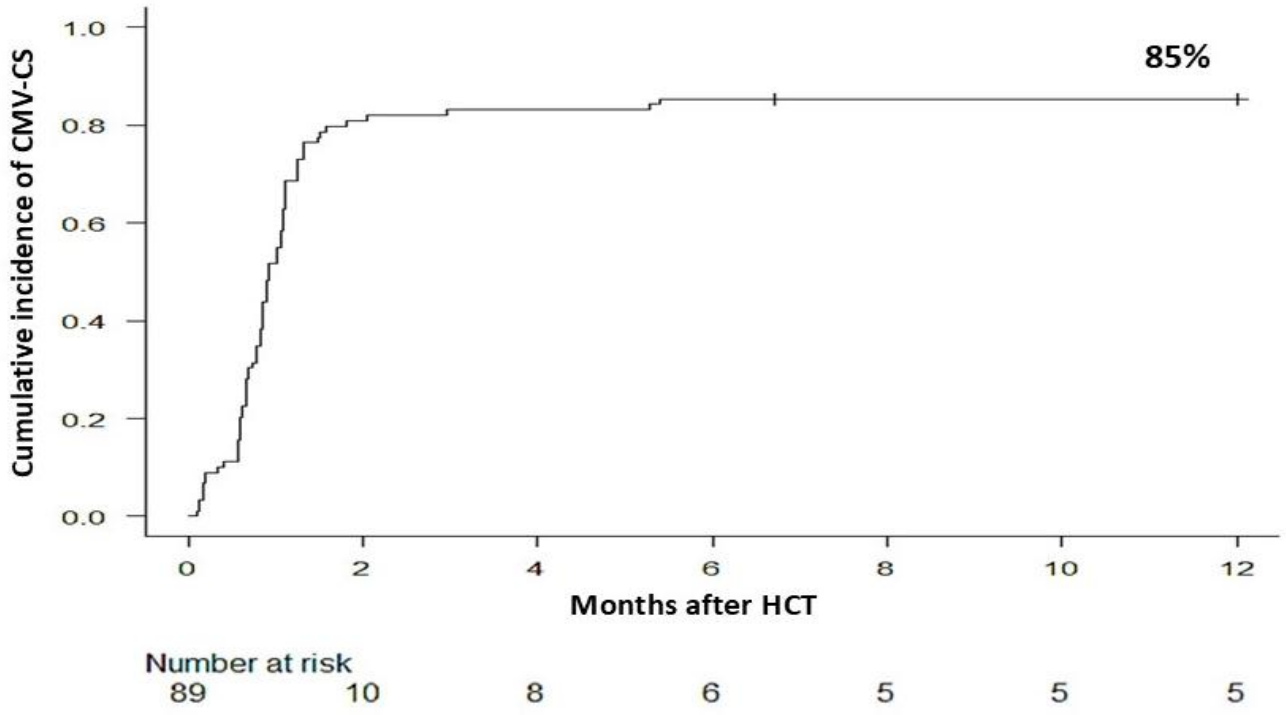


FIGURE 2 – Comparasion between patients with early CMV-CS and no early CMV-CS over

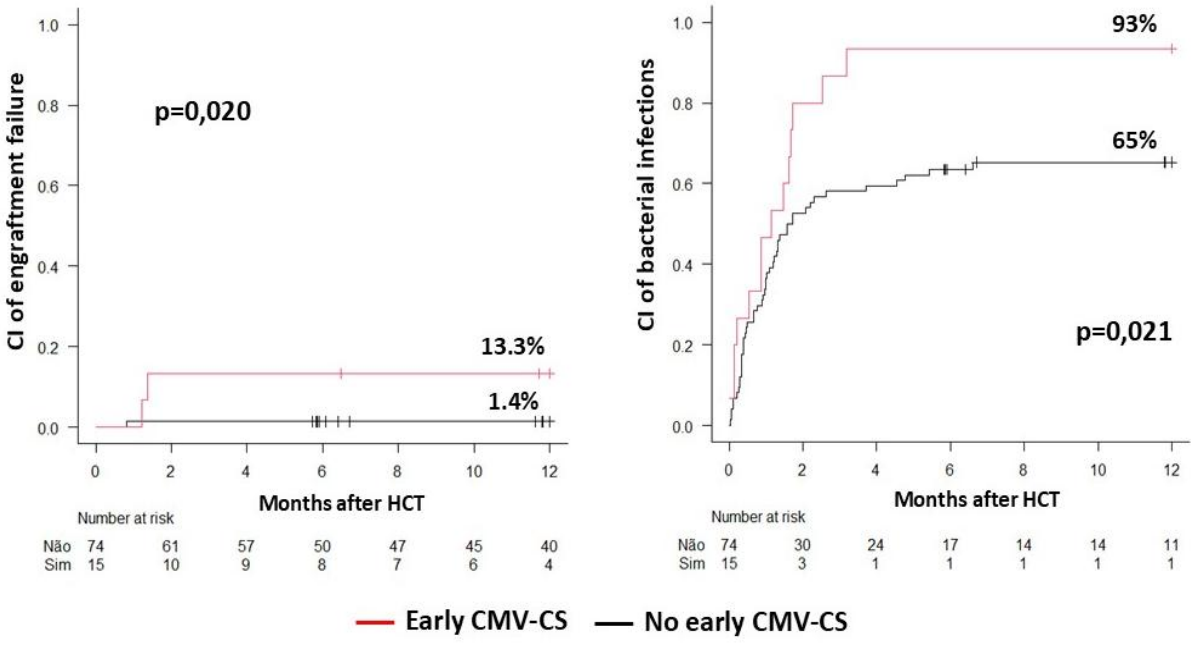
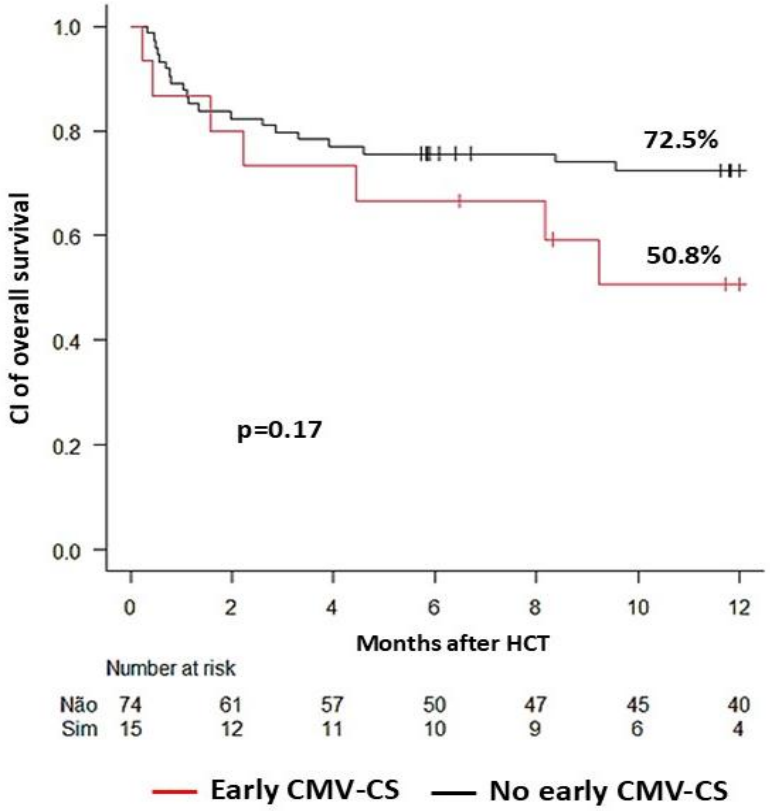


FIGURE 3 CI of overall survival - early CMV-CS and no early CMV-CS



INTRAVENOUS PENTAMIDINE AS PROPHYLAXIS FOR PNEUMOCYSTIS PNEUMONIA IN HEMATOLOGIC PATIENTS: A REAL-WORLD EXPERIENCE

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INTRODUCTION:

Pneumocystis jirovecii pneumonia (PJP) is a potentially life-threatening opportunistic infection in immunocompromised individuals, particularly patients with hematologic malignancies or undergoing hematopoietic stem cell transplantation (HSCT). While trimethoprim-sulfamethoxazole (TMP-SMX) remains the first-line prophylactic agent, its use is often limited due to intolerance or adverse effects. In such cases, alternative prophylactic strategies are necessary.

OBJECTIVE:

This study aims to describe the experience of a Brazilian private hospital with intravenous (IV) pentamidine as prophylaxis for PJP in hematologic patients.

METHODS:

We conducted a retrospective observational study including patients from January 2019 to December 2024 who received monthly IV pentamidine for PJP prophylaxis. Data were collected from electronic medical records and included demographics, underlying hematologic conditions, indication for PJP prophylaxis, prior adverse reactions to TMP-SMX, duration of pentamidine use, and occurrence of breakthrough PJP infections.

RESULTS:

A total of 39 patients were included, with a median age of 64 years (range: 3–84) and a male predominance (62%). The hematological diagnoses included acute leukemias (15-31%), lymphomas (11-28%), multiple myeloma (4-10%), myelodysplastic syndrome (3-8%), and others (8-18%). 29 patients underwent HSCT (20 allogeneic and 9 autologous) and 2 patients underwent CAR-T cell therapy. Prevention of worsening of myelosuppression / poor graft function was the primary reason for using pentamidine (24 – 61%) and TMP-SMX hypersensitivity reactions was the reason in the remaining patients. All adult patients received intravenous pentamidine at a dosage of 300 mg every four weeks, while pediatric patients were administered weight-adjusted doses of 3-4 mg/kg. The median number of doses was 3 (1-27), and most infusions was performed in an outpatient setting. Across the 39 patients analyzed, no confirmed cases of breakthrough PJP were observed. Adverse events were infrequent and generally mild, including headache (3%) and nausea (3%). No patient discontinued pentamidine due to toxicity. Also, no cases of toxoplasmosis were observed.

CONCLUSIONS

Intravenous pentamidine proved safe and effective for PJP prophylaxis in hematologic patients who couldn't tolerate TMP-SMX. No breakthrough infections occurred during the observation period. Its favorable safety profile and ease of administration support its continued use as a second-line agent in high-risk hematologic populations.

IS CLOSTRIDIOIDES DIFFICILE INFECTION A PREDICTOR OF ACUTE GRAFT-VERSUS-HOST DISEASE?

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INTRODUCTION:

Clostridioides difficile infection (CDI) is a major cause of acute diarrhea in allogeneic HSCT recipientes and is the leading infectious etiology in this setting. Its relationship with acute gastrointestinal graft-versus-host disease (GI-aGVHD) is under increasing investigation due to their potential bidirectional interaction.

OBJECTIVE:

To assess the association between CDI and GI-aGVHD in allogeneic HSCT recipients at a private hospital. Methods: Retrospective observational study of 11 patients (median age 48; range 33–68.5): 4 unrelated, 3 matched siblings, 4 haploidentical; 6 myeloablative conditioning, 5 reduced intensity conditioning; indications included acute myeloid leukemia (4), acute lymphoblastic leukemia (2), sickle cell disease (2), myelofibrosis (2), myelodysplastic syndrome (1). CDI diagnosis followed Infectious Diseases Society of America and Society for Healthcare Epidemiology of America criteria.

RESULTS:

Two patients (20%) developed GI-aGVHD, and two (20%) were diagnosed with CDI. Both GI-aGVHD cases occurred in related transplants (one haploidentical). GVHD prophylaxis included: PTCy + MMF + sirolimus (20%), PTCy + CsA + MMF (30%), CsA + MTX (30%), and CsA + MMF (20%). No specific regimen was associated with higher GVHD risk. One patient developed CDI before GI-aGVHD onset; the other did not have confirmed CDI. The odds ratio for association was 7.0 (95% CI: 0.22–226.02), suggesting a potential link without statistical significance due to sample size.

CONCLUSION:

Despite limited statistical power, findings suggest CDI may precede GI-aGVHD, indicating possible interaction. This underscores the importance of thorough etiologic investigation of post-transplant diarrhea to guide management. Larger studies are needed to clarify the causal relationship and clinical implications.

KEYWORDS: GvHD, Clostridioides, Transplant.

LATE INVASIVE PNEUMOCOCCAL DISEASE IN ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANT (HSCT) RECIPIENT: HOW CRUCIAL IS TO REMEMBER VACCINES

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INTRODUCTION:

Invasive pneumococcal disease (IPD) poses a significant risk for patients undergoing HSCT, particularly those with graft-versus-host disease (GVHD) and extended immunosuppressive therapy. Studies indicate a high incidence of IPD in transplant patients, especially later than 100 days post-transplant, with substantial intensive care unit admission and mortality rates surpassing 20%. In Brazil, data on post-HSCT IPD are limited, with few studies addressing antimicrobial resistance.

OBJECTIVE:

This report describes a case of an allogeneic HSCT recipient experiencing late IPD.

CASE REPORT:

A 26-year-old male diagnosed with early T-cell acute lymphoblastic leukemia in November 2023 underwent allogeneic HSCT from a matched sibling donor in August 2024 after achieving MRD negativity. His conditioning consisted of TBI and cyclophosphamide, with GVHD prophylaxis including cyclosporine and methotrexate, alongside infectious prophylaxis. Post-transplant complications included febrile neutropenia, CMV reactivation, and skin, liver, and gastrointestinal GVHD. A thrombotic microangiopathy developed, leading to adjustment of treatment. On day +204 post-transplant, he presented with fever, headache, and vomiting, later diagnosed with meningitis. Cerebrospinal fluid cultures revealed Streptococcus pneumoniae. After initial treatment with ceftriaxone, which was switched due to resistance, he was placed on linezolid. Despite some clinical improvement, he developed a brain abscess and required ongoing linezolid treatment alongside corticosteroid tapering. He was discharged after eight weeks of therapy.

DISCUSSION:

IPD is a serious threat to HSCT recipients. Reports indicate a late IPD incidence of 8.6 per 1000 transplants, with a mortality rate of around 20%, often linked to extensive corticosteroid use. While little data exists from Brazil, resistance to penicillin and ceftriaxone has been noted in serotypes linked to epidemic clones. This case illustrates the critical nature of ceftriaxone-resistant pneumococcal infections in immunocompromised patients, emphasizing the necessity for microbiological surveillance and early

detection of resistance. Pneumococcal vaccines like PCV13 and PPSV23 can lower IPD incidence in immunocompromised groups, but Brazilian vaccination coverage is inadequate, especially in non-priority adult populations. The recently recommended 20-valent pneumococcal conjugate vaccine (PCV20) offers a broader coverage of serotypes linked to invasive disease and should be considered for high-risk patients, including those undergoing HSCT.

CONCLUSION:

IPD remains a life-threatening complication for post-HSCT patients, especially with GVHD and prolonged immunosuppressive treatment. This case underscores the need for epidemiological monitoring, early detection of antimicrobial resistance, and the implementation of preventative strategies, including vaccination.

LEVOFLOXACIN AS ANTIBACTERIAL PROPHYLAXIS AND CLINICAL OUTCOMES IN A PRIVATE BONE MARROW TRANSPLANT CENTER

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INTRODUCTION:

Bacterial infections are frequent and potentially severe complications in bone marrow transplant (BMT), especially during neutropenia. Antibacterial prophylaxis is used to reduce infection incidence, but its use remains controversial due to concerns regarding resistance.

OBJECTIVE:

To evaluate outcomes of levofloxacin prophylaxis during neutropenia and describe bacterial infection patterns in patients undergoing BMT at a Brazilian private hospital over one year.

METHODS:

A retrospective observational study of 17 BMT patients (2024–2025), median age 47 (range 11–78). There were 11 allogeneic transplants (5 unrelated, 3 matched sibling, 3 haploidentical) and 6 autologous (3 for multiple myeloma, 3 for refractory lymphomas). Conditioning regimens included myeloablative (MAC, n=7) and reduced-intensity (RIC, n=4). Underlying diseases included acute myeloid leukemia (4), acute lymphoblastic leukemia (2), sickle cell disease (2), myelofibrosis (2), and myelodysplastic syndrome (1). Eleven patients received levofloxacin (750 mg/day) from the onset of aplasia to neutrophil recovery; six did not.

RESULTS:

Febrile neutropenia occurred in 14 patients; the three who remained afebrile received levofloxacin prophylaxis. Three bacterial infections were confirmed: two mucosal barrier-related (both with multisensitive organisms) and one skin infection. Antibiotics used included cefepime (n=11), meropenem (n=5), piperacillin-tazobactam (n=3), teicoplanin (n=3), vancomycin (n=1), and metronidazole (n=1). All isolates were sensitive to carbapenems, likely due to >90% adherence to hand hygiene protocols. Patients were placed in individual positive-pressure rooms with unidirectional airflow and HEPA filtration in a specialized BMT unit. Median time to engraftment was 14.7 days (range 10–21). All autologous transplant recipients used levofloxacin for less than 15 days. Transplant-related mortality was 0%, and all patients were alive at analysis with a median post-BMT survival of 240 days (range 25–457).

CONCLUSION:

Rational use of levofloxacin during neutropenia proved safe and effective, with low rates of severe infection, no multidrug resistant agent and no transplant-related mortality. Early infection management and adequate isolation infrastructure contributed to favorable outcomes.

KEYWORDS: Levofloxacin, Bacteria, Transplant.

LOW CORRELATION BETWEEN COLONIZATION AND BLOODSTREAM INFECTION BY RESISTANT PATHOGENS IN FEBRILE NEUTROPENIA EPISODES IN ONCO-HEMATOLOGIC PATIENTS: A RETROSPECTIVE STUDY INCLUDING CHEMOTHERAPY AND STEM CELL TRANSPLANTATION CONTEXTS

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Febrileneutropeniaisacommonandpotentiallysevere complication in immunocompromised patients, particularly those undergoing chemotherapy or hematopoietic stem cell transplantation. Although often managed as an infectious emergency, many episodes are not microbiologically documented and may result from non-infectious causes. In parallel, increasing concerns about multidrug-resistant organisms have led to empirical treatments guided by colonization status, despite the uncertain correlation between colonization and bloodstream infection. In this context, we aimed to determine the rate of microbiologically documented infections among febrile neutropenia episodes, describe the antimicrobial resistance profile of isolated pathogens, and assess the correlation between prior colonization and bloodstream infection. This retrospective study included 51 febrile neutropenia episodes occurring in 37 onco-hematological patients during 2024. Most episodes occurred during chemotherapy or hematopoietic stem cell transplantation (HSCT). Among the 37 patients, 21 underwent autologous HSCT (13 for multiple myeloma and 8 for lymphoma: 3 Hodgkin lymphoma and 5 non-Hodgkin lymphoma), 11 underwent allogeneic HSCT (for acute leukemias, myelodysplastic syndrome or sickle cell anemia), and 5 received chemotherapy without HSCT. Blood cultures were positive in 18 episodes (35.3%), with 22 pathogens identified. Gram-negative bacilli predominated (n=17), including Klebsiella pneumoniae (n=8; 2 KPC), Pseudomonas aeruginosa (n=3; all susceptible), Enterobacter cloacae (KPC), Escherichia coli (ESBL), and others. Fungal isolates included Candida tropicalis and Trichosporon asahii. Only 4 episodes involved multidrug-resistant (MDR) pathogens: 3 KPC-producing Enterobacterales and 1 ESBL-producing E. coli. Colonization by KPC-producing K. pneumoniae was present in 20 episodes, but bloodstream infection by the same organism occurred in only 2. Colonization by Enterobacter KPC occurred in 2 episodes without subsequent bacteremia by the same species. One patient colonized by both K. pneumoniae KPC and Enterobacter KPC developed polymicrobial bacteremia with K. pneumoniae KPC and Pseudocitrobacter faecalis KPC, the latter not previously detected. Colonization by ESBL-producing organisms occurred in 35 episodes. Only one led to bloodstream infection by the same pathogen with matching resistance; in 21, cultures were negative, and in 13, other pathogens were isolated (11 susceptible and 2 MDR, one with matching colonization). These findings highlight the limited predictive value of colonization status in guiding empirical therapy. Most MDR infections occurred in patients with prolonged hospital stays or other risk factors, with only one episode arising in a patient with no prior admissions.

KEYWORDS: febrile neutropenia, resistant pathogens, bloodstream infection

LOW RATE OF BACTEREMIA CAUSED BY MULTIDRUG-RESISTANT GRAM-NEGATIVE ORGANISMS IN PATIENTS PREVIOUSLY COLONIZED

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INTRODUCTION:

Infection (especially bacteremia) caused by multi-drug-resistant Gram-negative (MDRGN) bacteria is a serious problem in severely immunosuppressed patients such as those with hematologic malignancies. Previous colonization by MDRGN bacteria may predict the subsequent development of infection.

METHODS:

To evaluate the frequency of bacteremia caused by MDRGN bacteria in patients previously colonized by MDRGN bacteria, we retrospectively reviewed the charts of all patients admitted to a hematology unit with colonization by MDRGN bacteria between 2014 and April 2025. We reviewed all records of blood cultures obtained in the 6-month period after the documentation of colonization by MDRGN.

RESULTS:

We identified 69 patients colonized by MDRGN bacteria. The median age was 53 years old (range 17-89) and 59% were males. The most common underlying diseases were acute leukemia (30%) and non-Hodgkin's lymphoma (29%), and 36% were neutropenic at the time of documentation of colonization. The most frequent colonizing bacteria were Klebsiella pneumoniae (74%) and Escherichia coli (7%). Twenty patients did not develop any febrile episode and did not have blood cultures obtained. Among the remaining 49 patients with a febrile / infectious episode, 24 (49%) had positive blood cultures: 3 by Gram-positive bacteria and 21 by Gram-negative bacteria. Bacteremia by the same agent and susceptibility profile of the colonizing organism was observed in 6 patients only (8.6%). Age, gender, underlying disease, treatment, presence of neutropenia, mucositis, invasive procedures and time from colonization and bacteremia were similar in colonized patients with and without bacteremia by the same organism.

CONCLUSIONS:

The frequency of bacteremia caused by MDRGN bacteria among patients colonized by such organisms was low. No factor predictive of bacteremia was identified. The management of high-risk hematologic patients with colonization by MDRGN bacteria remains a great challenge.

MULTIDRUG-RESISTANT BACTERIA COLONIZATION AND BLOODSTREAM INFECTION PROFILE IN A HEMATOPOIETIC STEM CELL TRANSPLANTATION CENTER

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INTRODUCTION:

Infection is one of the main causes of death in Hematopoietic Stem Cell Transplant (HSCT) recipients. Outcomes improvement are correlated to infection prevention and control initiatives. Nevertheless, patients in the setting of HSCT are at high risk of developing bacterial infections due to prolonged neutropenia and the emergency of multidrug-resistant (MDR) Enterobacteriaceae, Pseudomonas aeruginosa and vancomycin-resistant Enterococcus (VRE) whose bacteremia are associated to high mortality rates.

OBJECTIVES:

This study´s purpose is to assess MDR colonization prevalence and bloodstream infection (BSI) and impact in clinical outcomes in autologous HSCT patients.

METHODS:

This is a single center, transversal study that retrospectively analysed data from 17 patients from September 2024 to May 2025. It evaluated the pre engraftment colonization profile, BSI rate and clinical outcomes. Surveillance swabs were weekly collected from multiple sites (nasal, perirectal and axillary) since patient admission until its discharge, and cultured on chromogenic selective media. Blood cultures were collected during febrile events and analysed by mass spectometry (ViteK2).

RESULTS:

The main indications for autologous HSCT was multiple myeloma (64,70%) and non-Hodgkin Lymphoma represented the rest (35,3%). Median age was 64 years. One patient presented with pre engrafment surveillance swab positive por Carbapenemase-producing Klebsiella pneumoniae (KPC). A total of 29,41% of patients had bloodstrean infections. The most prevalente strain was Klebsiella pneumoniae sp acounting for 60% of BSI. Other bacteria found was Enterobacter cloaceae, Streptococcus mitis, staphylococcus epidermidis and Pantoea . The patient formerly colonized by KPC developed a non ESBL/MDR Klebsiella pneumoniae BSI. In first febrile neutropenia event, Cefepime was chosen in 62,5% of patients; Piperacillin-Tazobactam, in 1 patient (6,25%) and association of Meropenem and teicoplanin, in 31,25% of patients due to hemodynamic instability. Only 1 patient had teicoplanin associated to Cefepime in first febrile event due to suspected catheter infection, not confirmed later. The median duration of antibiotic use in first febrile event when cultures remained negative was 6 days. The median time to neutrophil

engraftment was 11 days. There was no primary graft failure. The mortality rate D+30 was 6,25% (1 patient) due to sepsis after engrafment with BSI due to Pantoea. The Median time to discharge was 19 days.

CONCLUSION:

We found a low rate of pre engrafment colonization to MDR bacteria. The multidisciplinary microbial stewardship includes the rational antibiotic use guided by screening swabs and blood cultures as importante strategies to overcome the challenge of preventing BSI and reduce early mortality in HSCT recipients.

KEYWORDS: Hematopoietic Stem Cell Transplantation, Febrile Neutropenia, Antimicrobial Stewardship

NOCARDIA PULMONARY INFECTION IN AN IMMUNOCOMPROMISED PATIENT: A CASE REPORT AIMED TO CREATE AWARENESS

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INTRODUCTION:

Nocardiosis is an uncommon, yet severe, opportunistic infection primarily caused by members of the Nocardia genus that affects immunocompromised hosts, such as haematopoietic stem cell or solid organ transplantation recipients and patients treated with long-term immunosuppressive agents or high-dose corticosteroid therapy.

CASE REPORT:

A 51-year-old female patient diagnosed with high-risk myelodysplastic syndrome with excess blast 1 (MDS-EB1) underwent first-line treatment with a matched related donor allogeneic HCT. The conditioning regimen consisted of fludarabine (30 mg/m^2, 5 days) and intravenous (IV) busulfan (3.2 mg/kg, 4 days). GVHD prophylaxis consisted of post-cyclophosphamide and cyclosporine. One year after transplantation, the patient developed grade III lung and liver chronic graft-versus-host disease (cGVHD), managed with systemic corticosteroids and, alter no response, ruxolitinib was prescribed, which produced a good response. After three months, the patient presented to the emergency department with fever, cough, dyspnea and pleuritic pain on the left. Empirical antibiotic therapy was started and as the chest CT scan showed a subpleural pulmonary consolidation in the left posterior basal segment with small central cavitation, the antifungal voriconazole was associated. A percutaneous biopsy was performed and the culture of the material showed growth of Nocardia otitidiscaviarum and the antibiotic therapy was shifted according to antibiotic susceptibility testing to linezolid (600mg IV every 12 h) plus TMP-SMX (15mg/kg/day IV, divided into four doses) and continued for almost 6 weeks. Follow-up imaging showed progressive improvement in the lesions. The patient is still on treatment and undergoing periodic clinical monitoring, with also improvement in the respiratory functional tests.

DISCUSSION:

Nocardia species are ubiquitous, filamentous, aerobic, Gram-positive, partially acid-fast bacilli that can cause localized or disseminated disease, most commonly affecting the lungs, skin and brain. Nocardia can cause severe morbidity and mortality, particularly in patients with comorbidities or compromised immunity. Opportunistic infections in immunocompromised patients can present a diagnostic challenge, leading to delayed diagnosis and treatment. Risk of concomitant infection such as aspergillosis also presents a risk.

CONCLUSION:

This case report demonstrates the importance of lung biopsy in the correct diagnosis and bring awareness about nocardiosis as it should be suspected in cases of infection affecting skin, soft tissue, musculoskeletal, respiratory and central nervous system. Also diagnostic difficulties and lack of antibiotic susceptibility tests for Nocardia spp. can delay appropriate treatment and lead to unfavorable outcomes.

POST-TRANSPLANT VIRAL INFECTIONS IN BONE MARROW RECIPIENTS: REAL-WORLD DATA FROM A PRIVATE CENTER

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INTRODUCTION:

Despite prophylaxis and preemptive therapies, viral infections remain highly prevalent after bone marrow transplantation (BMT) and contribute to non-relapse mortality.

OBJECTIVE:

Evaluate the incidence of viral infections and one-year outcomes in patients undergoing BMT in a private hospital.

METHOD:

This is a retrospective study including 17 patients, median age 47 years (range 11–78). Among them, 11 underwent allogeneic BMT (5 unrelated, 3 matched related, 3 haploidentical); 7 received myeloablative conditioning (MAC) and 4 reduced-intensity conditioning (RIC), indicated for acute myeloid leukemia (4), acute lymphoblastic leukemia (2), sickle cell anemia (2), myelofibrosis (2) and myelodysplastic neoplasm (1). Six patients underwent autologous transplants (3 for multiple myeloma, 3 for refractory lymphoma as second-line therapy). All patients received antiviral prophylaxis with valacyclovir 500 mg every 12h or acyclovir 400 mg every 12h from the beginning of conditioning (if not already on prophylaxis), continued for 1 year post-transplant. Three high-risk patients also received CMV reactivation prophylaxis with letermovir. All patients were in protected environment rooms in a dedicated BMT unit, with individual rooms, positive air pressure with continuous monitoring, unidirectional airflow, and HEPA filtered air. In suspected respiratory viral infection, patients were transferred to negative-pressure rooms until symptoms resolved or respiratory panel ruled out viral infection.

RESULTS:

Median time to engraftment was 14.7 days (range 10–21). Of the 17 patients, 5 had at least one viral infection (overall incidence 29.4%; 45.4% among allogeneic BMT patients). Of these 5, 4 had cytomegalovirus (CMV) reactivation—an incidence of 36.3% among allogeneic BMT patients. All received preemptive therapy with ganciclovir; one was switched to maribavir due to myelotoxicity. All achieved viral clearance after treatment. One patient developed dengue and rotavirus infections, with full symptom resolution after supportive care. One haploidentical transplant patient had parainfluenza 3 respiratory infection (mild symptoms) and adenovirus reactivation, progressing to grade 3 hemorrhagic cystitis. Treatment included IV cidofovir (5 mg/kg and 3 mg/kg) and intravesical cidofovir (2 doses of 5 mg/kg), with significant viral load reduction and improved hematuria. Of the 17 patients, only one died due to acute myeloid leukemia relapse, resulting in 0% transplant-related mortality.

CONCLUSION:

Overall viral infection rates were consistent with the literature, including a relatively low CMV reactivation rate (36.3%) among allogeneic BMT patients, and no cases of CMV disease. No transplant-related deaths occurred, even in the presence of adenovirus reactivation, which is associated with high mortality. These findings underscore the importance of a protective environment, along with prophylaxis and preemptive therapy strategies in BMT patients.

KEYWORDS: Virus, CMV, Transplant.

RARE ONCO-HEMATOLOGY CASE: T(8;21) AML WITH GRANULOCYTIC SARCOMA AND TUBERCULOSIS SUCCESSFULLY TREATED WITH TARGETED THERAPY AND HSCT

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A 54-year-old female patient was diagnosed with acute myeloid leukemia (AML) with t(8;21) translocation and NRAS mutation. Initially treated with the "7+3" induction regimen combined with Mylotarg, she showed refractory disease. She presented with persistent fever and progressively worsening axillary lymphadenopathy. An excisional biopsy initially revealed granulocytic sarcoma, prompting a change in therapy to venetoclax + azacitidine. However, subsequent histopathological reports identified concurrent tuberculous lymphadenitis, leading to the initiation of COXCIP 4 regimen alongside oncologic treatment. Therapeutic management required careful monitoring and adjustments, including dose reduction of venetoclax due to drug interactions. After completing three full cycles of the combined therapy, the patient achieved measurable residual disease (MRD)-negative status. She then underwent allogeneic hematopoietic stem cell transplantation (HSCT) with a full-matched sibling donor (HLA-identical, CMV serology-matched). The conditioning regimen used was Bu3Flu5, with graft-versus-host disease (GVHD) prophylaxis consisting of cyclosporine and methotrexate. Cryopreserved hematopoietic stem cells were infused on July 6, 2023, with neutrophil recovery documented by day +16 (July 22, 2023). Post-transplant follow-up included nine months of antituberculosis treatment, with excellent adherence and tolerability. Currently, at two years post-transplant, the patient remains in complete hematologic remission with MRD-negative status and full donor chimerism, along with successful resolution of tuberculosis. This case highlights the complexity of managing concurrent AML and tuberculosis in an immunocompromised setting, emphasizing the importance of a multidisciplinary and individualized approach. The combination of venetoclax and azacitidine proved effective despite requiring dose adjustments, enabling disease control and subsequent successful allogeneic transplantation. The co-occurrence of granulocytic sarcoma and tuberculous lymphadenitis represents a particularly rare finding in the literature, underscoring the need for active surveillance of concurrent diagnoses in AML patients. The favorable long-term outcome—marked by durable remission at two years—demonstrates the feasibility and effectiveness of this tailored therapeutic strategy in complex clinical settings.

KEYWORDS:

Acute Myeloid Leukemia (AML), Tuberculous Lymphadenitis, Allogeneic Hematopoietic Stem Cell Transplantation (HSCT), granulocytic sarcoma

RETROSPECTIVE EVALUATION OF THE SEPSIS MANAGEMENT PROTOCOL IN PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION IN A TERTIARY PUBLIC HOSPITAL (2019-2024)

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INTRODUCTION:

Patients undergoing hematopoietic stem cell transplantation (HSCT) have a high susceptibility to sepsis due to profound immunosuppression caused by myeloablative conditioning, post-transplant immune dysfunction, and complications such as graft-versus-host disease (GVHD). Rapid identification of clinical and laboratory signs of sepsis, followed by immediate implementation of standardized therapeutic measures, is essential to mitigate the exaggerated inflammatory response and reduce morbidity and mortality in this high-risk immunocompromised population.

OBJECTIVE:

To retrospectively evaluate adherence to and effectiveness of the sepsis management protocol in patients undergoing hematopoietic stem cell transplantation (HSCT) in a tertiary public hospital, identifying factors associated with early detection, interventions performed, and clinical outcomes.

METHOD:

A retrospective observational study was conducted analyzing data from patients undergoing HSCT diagnosed with sepsis between 2019 and 2024 at HTEJZ. Data collection was performed through review of electronic medical records, sepsis forms completed by the clinical team, and associated laboratory tests. Information was organized in Microsoft Excel spreadsheets for subsequent analysis. Sepsis management was carried out according to an institutional protocol based on guidelines from the Latin American Sepsis Institute (ILAS). Adherence to and effectiveness of this protocol were evaluated. Results: Sixty-one patients were included, predominantly male (57%) with a median age of 61 years. Most patients (80%) underwent autologous transplantation. The prevalent infectious diagnosis was sepsis (82%), followed by septic shock (18%). The infectious origin was mostly hospital-acquired (87%), with pulmonary and abdominal foci being the most frequent. Adherence to the first-hour therapeutic bundle was observed in 84% of cases. Among patients with positive cultures (32%), microorganisms such as Klebsiella pneumoniae, Clostridium difficile, Enterococcus spp., and Staphylococcus epidermidis stood out. Positive cultures were more common in sepsis cases without shock. The outcome was favorable (hospital discharge) in 85% of patients. Death occurred in 9 cases (15%), more frequently among patients undergoing allogeneic transplantation with intense myeloablative conditioning and abdominal or pulmonary focus. Non-adherence to the first-hour bundle was associated with worse outcomes.

CONCLUSION:

Patients undergoing hematopoietic stem cell transplantation who develop sepsis, especially after allogeneic transplantation and with abdominal or pulmonary foci, have a higher risk of complications and mortality. Adherence to the first-hour bundle was associated with better outcomes, highlighting the importance of rapid identification and intervention against infections in this immunocompromised patient profile.

KEYWORDS:

hematopoietic stem cell transplantation; sepsis; Protocol Adherence

SURVIVING ASPERGILLUS ENDOCARDITIS AND MENINGITIS AFTER HEMATOPOIETIC STEM CELL TRANSPLANT (HCT)

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INTRODUCTION:

Aspergillus spp can cause invasive infections in several organs, commonly in the lungs and sinuses, but also in organs such as the heart and central nervous system.

OBJECTIVE:

To present the strategy treating a patient who survived an Aspergillus endocarditis and meningitis. Method: Review of the electronic medical records.

RESULT:

A 13-year-old boy underwent HCT for the treatment of T-cell acute lymphoblastic leukemia with primary induction failure. Two and a half years after the transplant, while on immunosuppression for the treatment of chronic graft-versus-host disease and presented with fever that did not respond to broad spectrum antibiotics. Within the following week he developed a skin nodule and decreased consciousness. Liposomal amphotericin was started promptly but Aspergillus was identified in the skin biopsy and in the cerebrospinal fluid. Voriconazole was associated but a vegetation in the mitral valve was shown in the echocardiogram. After 4 weeks of combined therapy, he underwent surgical removal of the cardiac vegetation. Pathology showed many fungal forms suggestive of hyalohyphomycosis. The patient completed 8 weeks of amphotericin, and the subsequent echocardiogram and cerebrospinal fluid showed no signs of infection and will remain on voriconazole until discontinuation of all immunosuppression.

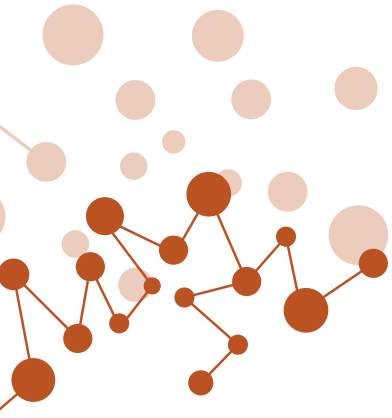
DISCUSSION:

Aspergillus infection with endocarditis and meningitis are rare forms of the disease, with a poor prognosis, which require to receive appropriate treatment. For the adequate treatment of Aspergillus endocarditis, in most cases, it is necessary to use antifungal agents combined with surgical debridement.

CONCLUSION:

Severe fungal infections may be successfully treated if the team has a high suspicion, a quick diagnosis is made, aggressive and appropriate treatment are offered. Fungal endocarditis also requires aggressive surgical debridement by a very experienced team.

KEYWORDS: Aspergillus; fungal endocarditis; fungal meningitis.



NON INFECTIOUS COMPLICATIONS

CASE SERIES OF HEMOPHAGOCYTIC LYMPHOHYSTIOCYTOSIS IN ADULTS IN A SINGLE INSTITUTION IN BRAZIL.

Miguel Pedro de Queiroz Neto[1]; Ana Paula de Azambuja[1]; Elenaide Coutinho Nunes[1]; Ricardo Pasquini[1]; Vaneuza Araújo Moreira Funke[1]

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INTRODUCTION

Hemophagocytic Lymphohystiocytosis (HLH) is a hyperinflamatory syndrome associated with excessive immune activation of macrophages, cytotoxic T lymphocytes (CTL) and natural killer cells (NK), which results in irreversible multiorgan damage and death. In adults, HLH is rare and commonly associated with infections, malignancies, CAR T-cell therapy, and hematopoietic stem cell transplantation. Despite published guidelines, HLH remains underdiagnosed due to overlapping features with other conditions, limited access to specific diagnostic tests and lack of epidemiological data in Brazil.

RESULTS

We present a case series of eight adult patients diagnosed and treated for HLH at our institution since 2019. Major clinical characteristics are depicted in table 1. Other features were elevated liver enzymes (8/8), fever (6/8), anemia (6/8), thrombocytopenia (5/8), leukopenia (3/8), hepatosplenomegaly (4/8), altered mental status (3/8), active bleeding (1/8) and nephrotic syndrome (1/8). Four patients had high EBV viral load, including one case of EBV-related post-transplant lymphoproliferative disorder and one case with also CMV reactivation. Other triggers included disseminated Histoplasmosis, ALK-negative Anaplastic Large T-cell Lymphoma, Extranodal NK/T-cell Lymphoma, and Chronic Myelomonocytic Leukemia. One patient developed HLH after CAR T-cell therapy (tisa-cel) for refractory Diffuse Large B-cell Lymphoma. All patients had hemophagocytosis on bone marrow evaluation, with markedly decreased or absent CTL and NK cells by flow cytometry. Six patients died from HLH-related complications. Initial treatment included corticosteroids and Immunoglobulins, with therapy subsequently tailored to the identified trigger. Two patients received Anakinra and five received Etoposide.

DISCUSSION

In our experience, HLH typically presents with cytopenia, fever, AST-predominant liver enzyme elevation, hyperbilirubinemia, hyperferritinemia and hepatosplenomegaly. Based on these findings, we developed a local diagnostic algorithm (Figure 1). The sCD25 test, although useful, is not part of our institutional routine due to cost constraints. Therefore, we adopt a ferritin threshold of ≥5,000 ng/mL to increase diagnostic sensitivity in the absence of sCD25, while acknowledging that other conditions can elevate it.

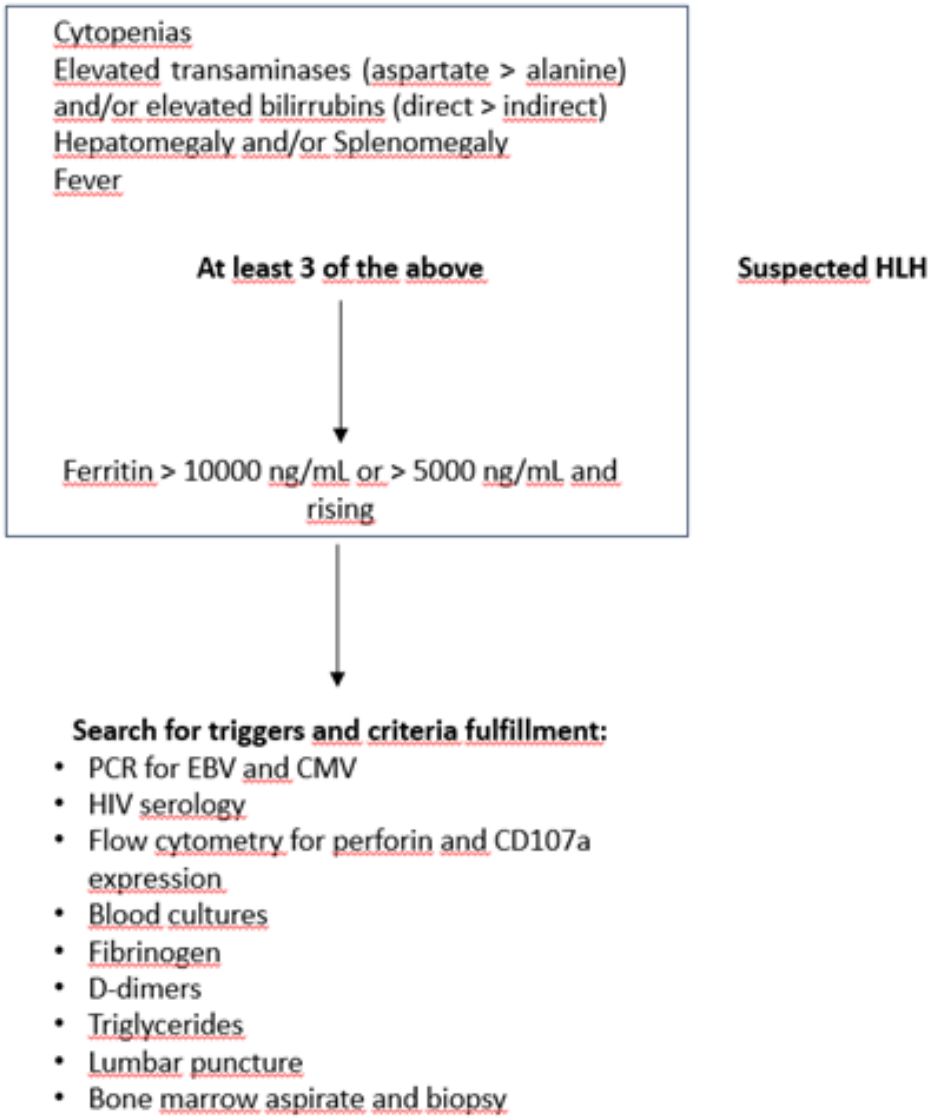
CONCLUSION

HLH is a rapidly progressive and underrecognized condition associated with high mortality. Inadequate access to diagnostic tests like sCD25 and viral PCRs hinders early recognition, especially in resource-limited settings such as Brazil. We favor the HScore over HLH-2004 criteria for diagnosis. Flow cytometry may offer a practical alternative for supporting diagnosis. Etoposide remains the most established treatment for refractory HLH but carries significant infection risk. Anakinra is a promising and safer alternative. Our findings reinforce the urgent need for broader access to more effective diagnostics and therapeutics strategies to HLH.

TABLE 1. Variables and median values of interest in our cohort.

Variables	Median Values (Range)	Thresholds
Age	55 years-old (18-84)	-
Ferritin	60793 ng/mL (8882-100000)	< 5000 ng/mL
EBV viral load	351752 copies/mL (635-1185000)	< 500 copies/mL
HScore at diagnosis	217 points (158-306)	< 135 points
AST / ALT	201 U/L (77-1442) / 144 U/L (57-1438)	< 30 U/L
Total bilirubin / direct bilirubin	6,4 mg/dL (0,7-9,9) / 4,7 mg/dL (0,3-6,5)	< 2,0 mg/dl / < 1,0 mg/dl
Absolute T-cell lymphocyte count	130 cells/mm^3 (15-4572)	700 – 2100 cells/mm^3
Absolute NK cells count	9.9 cells/mm^3 (0-171)	90 – 600 cells/mm^3
Fibrinogen	mg/dL (86-292)	>150mg/dL
Triglycerides	118mg/dL (84-503)	< 150mg/dL

FIGURE 1. Diagnostic approach in our center.



DRAMATIC CASE OF IDIOPATHIC PNEUMONIA SYNDROME WITH BILATERAL SPONTANEOUS PNEUMOTHORAX AND TENSION PNEUMOMEDIASTINUM.

Wysterlânyo Kayo Pereira Barros[1], Lucas Tejo Pereira de Brito Silva[1], Samuel de Sousa Custódio[1], Paloma Martinho Resende[1], André Costa Meireles[1], Renata Leati Stanzione[1], Mariana Nassif Kerbauy[1], Andreza Alice Feitosa Ribeiro[1], Nelson Hamerschlak[1]

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INTRODUCTION:

HSCT is a curative therapy for various hematologic malignancies; however, its success can be limited by complications, including pulmonary complications.

OBJECTIVES:

To report a case of idiopathic pneumonia syndrome (IPS), highlighting the importance of its recognition and the prognostic impact of this condition.

CASE REPORT:

A 55-year-old male patient diagnosed with AML underwent allogeneic HSCT after achieving complete remission following seven cycles of the Viale-C protocol. He received myeloablative conditioning with Bu4Flu combined with 2 Gy TBI, followed by haploidentical HSCT with peripheral blood stem cells. GVHD prophylaxis included cyclosporine, mycophenolate, and PTCy. In the early post-transplant period, the patient developed haplo fever, neutropenic colitis, and acute pulmonary edema, requiring transfer to the ICU and orotracheal intubation on D+16, followed by extubation seven days later. On D+27, the patient experienced new respiratory deterioration requiring reintubation. After extensive investigation, including bronchoalveolar lavage, the hypothesis of IPS was raised, leading to the initiation of methylprednisolone and ruxolitinib, with slight clinical improvement. At that time, the patient had no signs of GVHD, and bone marrow reassessment on D+30 showed negative measurable residual disease and full donor chimerism. On D+34, the patient experienced further respiratory worsening associated with extensive subcutaneous emphysema. Chest CT revealed a spontaneous right-sided pneumothorax, which required chest drainage. Two days later, he developed a contralateral pneumothorax, also requiring intervention. In the following days, the patient presented with progressive respiratory deterioration, worsening subcutaneous emphysema, and hemodynamic instability. A chest CT performed on D+44 demonstrated a tension pneumomediastinum, requiring emergent bedside drainage. Given the refractoriness of the clinical condition and in accordance with the family's wishes, palliative care measures were initiated on D+45. The patient passed away on D+47.

DISCUSSION:

The term IPS refers to a spectrum of diffuse pulmonary injuries. Its diagnosis involves evidence of diffuse alveolar injury accompanied by signs and symptoms of pneumonia, in the absence of active respiratory infection. IPS can occur in 4% to 12% of HSCT recipients, with a mortality rate ranging from 60% to 86% within the first 100 to 120 days post-transplant. Risk factors include myeloablative conditioning, high-dose TBI, severe acute GVHD, advanced age, and transplantation for MDS/AML.

Historically, management has involved high-dose corticosteroids, and more recently, etanercept (anti-TNF-α). In addition, case series have shown favorable outcomes with the use of ruxolitinib. Conclusion: This case highlights the importance of this severe post-transplant complication and its high mortality, as well as the need for a better understanding of its pathophysiology and the development of more effective therapies with a greater prognostic impact.

KEYWORDS:

Idiopathic pneumonia syndrome, Hematopoietic stem-cell transplantation (HSCT), Pulmonary complication.

FIGURE 1. Chest CT on D+27 showing bilateral ground-glass opacities, predominantly central, with areas of traction-like appearance.

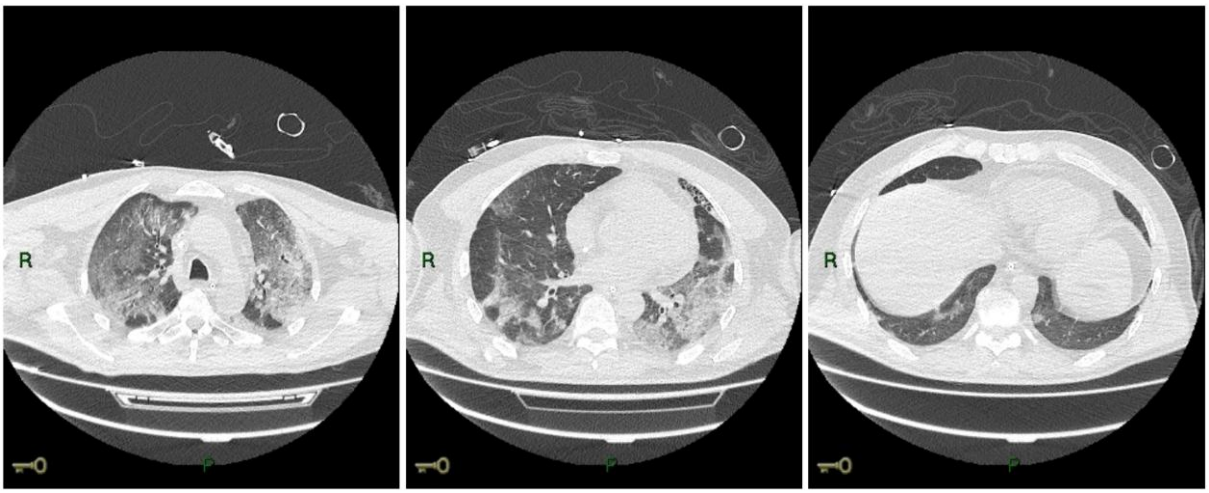
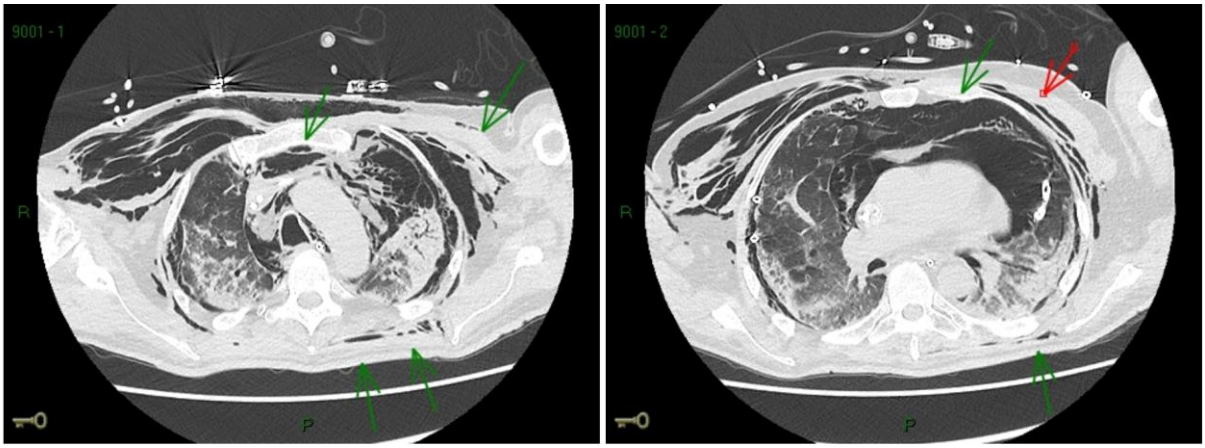


FIGURE 2. Chest CT on D+44 showing extensive mediastinal emphysema (causing posterior displacement of the heart) and subcutaneous emphysema along the entire thoracic wall.



MULTICOHORT TRANSCRIPTOME ANALYSIS REVEALS KEY GENES FOR SYSTEMIC LUPUS ERYTHEMATOSUS

Felipe Pantoja Mesquita[1,3], Thiago Loreto Matos[3], Marília Silveira Maia[1], Elizabeth De Francesco Daher[3], Pastora Maria Araújo Duarte[3], Fernando Barroso Duarte[1,2,3].

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3. Universidade Federal do Ceará, Fortaleza – CE - Brasil

INTRODUCTION:

Systemic lupus erythematosus (SLE) is a heterogeneous autoimmune disease with complex pathophysiology and limited therapeutic options. The absence of reliable patient stratification and robust biomarkers hinders precision medicine approaches in SLE.

AIM:

Therefore, this study aimed to identify consistently differentially expressed genes (DEGs) across independent transcriptome datasets from whole blood of SLE patients using a multicohort integrative strategy.

METHODS:

Public RNA-seq datasets (GSE72509, GSE112087, GSE122459, and GSE80183) were retrieved from the Gene Expression Omnibus. Batch effects were corrected using the ComBat_seq function from the sva package. DEG analysis was performed using both the limma+edgeR and DESeq2 frameworks, and only genes identified as significant in both approaches were retained. DEGs were selected based on a false discovery rate (FDR) < 0.01 and absolute log2 fold-change > 1. Gene Ontology (GO) enrichment analysis was conducted separately for upregulated and downregulated genes passing these criteria to characterize the biological processes most affected in SLE.

RESULTS:

First, the integrated transcriptomic analysis of multiple cohorts was corrected as described (Fig. 1). The corrected dataset revealed a robust set of differentially expressed genes between systemic lupus erythematosus (SLE) patients and healthy controls. The volcano plot (Fig. 1A) highlighted a prominent inflammatory signature strongly associated with innate immune activation, with upregulated genes such as IFI27, OTOF, IFI44L, SIGLEC1, RSAD2 (log2FC > 3, FDR < 0.01). These genes are primarily involved in the type I interferon signaling pathway. Functional enrichment analysis (Fig. 2B) showed that the differentially expressed genes were significantly associated with biological processes such as defense response to viruses, response to viruses, and negative regulation of viral genome replication, all GO related to interferon-signaling pathways.

CONCLUSION:

Our multicohort transcriptomic analysis highlights the pivotal role of type I interferon signaling and innate immune activation in the pathogenesis of systemic lupus erythematosus (SLE). The consistent upregulation of interferon-stimulated genes across independent datasets underscores their potential as robust biomarkers of disease activity and promising therapeutic targets. These findings deepen our understanding of SLE's molecular landscape and support the rationale for developing targeted therapies, including CAR-T cell strategies that aim to modulate aberrant immune responses.

KEYWORDS:

systemic lupus erythematosus, transcriptome, genes.

FIGURE 1 - **Batch effect correction across SLE transcriptomic cohorts.**

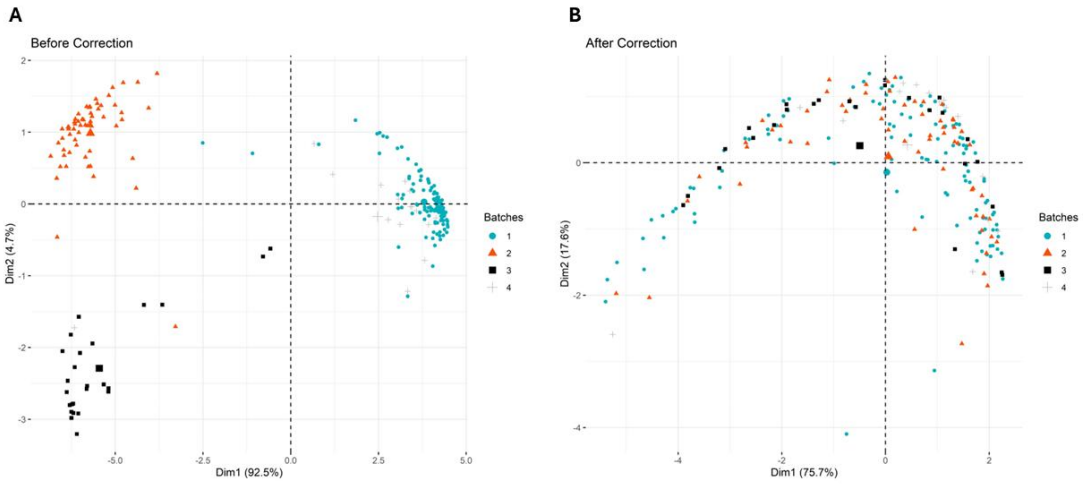
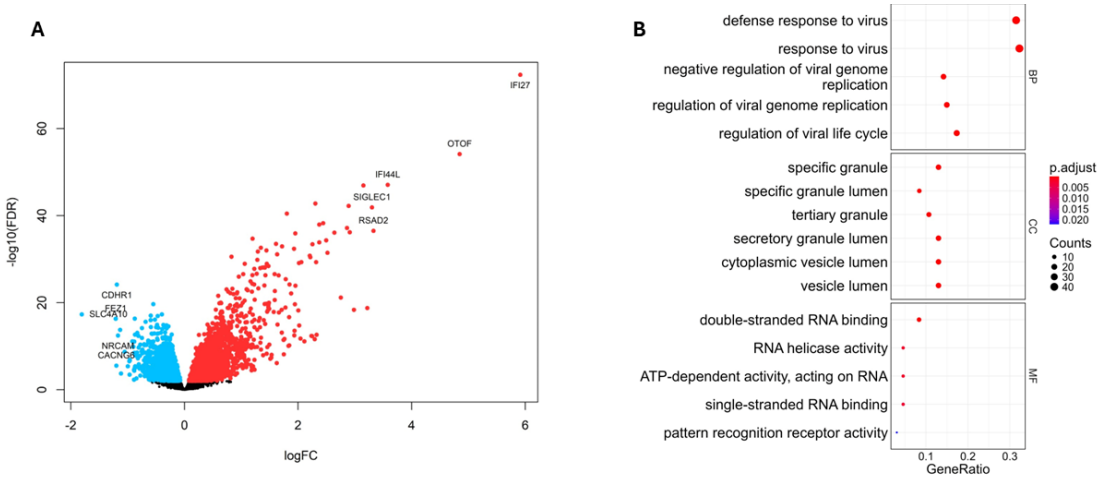


FIGURE 2 - **Differential gene expression analysis and Functional enrichment analysis by GO in systemic lupus erythematosus (SLE).**



PITUITARY APOPLEXY FOLLOWING ALLOGENEIC TRANSPLANTATION IN A PATIENT WITH ACUTE MYELOID LEUKEMIA SECONDARY TO HYPOPLASTIC MYELODYSPLASTIC SYNDROME: CASE REPORT

Talita Carvalho Siqueira[1], Nyara Walleska Maciel de França[1], Carla Campos Miranda[1], Mathias Regis Modesto[1], Ricardo Romano[1], Guilherme Cecchetti[1], Silvio Henrique Vieira Freitas[1], Marcos Roberto Curcio Pereira[1], Gabriela UgioniDuarte[1], Guilherme Lourenço[1], Ediely Layana Oliveira Coletto[1], Ana Paula de Azambuja[1], Rafael Marchesini[1], Felipe Borges Patrício[1], Glaucia Tagliari[1], Miguel Pedro de Queiros Neto[1], Samir K. Nabhan[1], Daniela Setubal[1], Caroline Bonamin Sola[1], Vaneuza Araújo Moreira Funke[1]

1. Bone Marrow Transplant Service of the Clinical Hospital Complex of the Federal University of Paraná (CHC-UFPR)

INTRODUCTION

Pituitary apoplexy is a neuroendocrine emergency caused by infarction or hemorrhage of previously undiagnosed pituitary adenomas. It may be triggered by infections, hypotension, anticoagulation, chemotherapy, or immunosuppression. In the context of hematopoietic stem cell transplantation (HSCT), especially following myeloablative conditioning and GVHD prophylaxis, vascular and metabolic stress increase the risk. Early recognition is essential to avoid permanent neurological and endocrine damage.

OBJECTIVE

To describe a rare case of hemorrhagic pituitary apoplexy after allogeneic HSCT in a patient with secondary acute myeloid leukemia (AML), emphasizing diagnostic and therapeutic aspects

METHODS

Descriptive case report based on clinical, laboratory, imaging, and therapeutic data from a patient with hypoplastic myelodysplastic syndrome who progressed to AML and underwent unrelated-donor HSCT. Clinical evolution, imaging findings (CT and MRI), endocrine assessments, and treatment response were reviewed. A literature review was performed using PUBMED, MEDLINE, and Science Direct, focusing on pituitary apoplexy and endocrine complications in HSCT recipients.

RESULTS

A 63-year-old male with transfusion-dependent hypoplastic MDS had 3.8% marrow blasts, multilineage dysplasia, and normal karyotype. He progressed to AML with 30% blasts and received three cycles of venetoclax plus cytarabine (VIALE-C), achieving MRD-positive remission. As consolidation, he underwent allogeneic HSCT from an unrelated donor with 11/12 HLA match. The conditioning regimen was myeloablative, using busulfan, fludarabine, and thymoglobulin. GVHD prophylaxis included cyclosporine and methotrexate.

During hospitalization, he developed Enterococcus faecium bacteremia, anorexia, and malnutrition requiring parenteral nutrition. On day +25, he presented with sudden neurological deterioration (confusion, agitation, desaturation, decreased consciousness), followed by cardiorespiratory arrest, reversed after one CPR cycle. Cranial CT revealed sellar hemorrhage. MRI showed a 3.1 × 2.5 × 1.8 cm pituitary macroadenoma with hyperintense T1/T2 signal,

no central enhancement, and bilateral cavernous sinus invasion (Knosp II), consistent with hemorrhagic apoplexy. High-dose IV hydrocortisone was started, then tapered to oral prednisone. Endocrine tests confirmed panhypopituitarism and adrenal insufficiency, and full hormonal replacement was initiated. Ophthalmologic and neurosurgical evaluations ruled out surgical need. The patient improved clinically and neurologically and remains stable on outpatient follow-up.

CONCLUSION

This case highlights a rare but serious post-HSCT complication. While surgery may be necessary in some cases, early corticosteroid therapy may prevent deterioration. Rapid recognition, multidisciplinary care, and hormonal replacement were key to recovery. Acute neurological decline in HSCT patients should prompt evaluation for endocrine emergencies such as pituitary apoplexy.

KEYWORDS:

pituitary apoplexy; hematopoietic stem cell transplantation; neuroendocrine emergency; MRI brain findings

SERUM ANGIOTENSIN 1-7 LEVELS AS A PREDICTIVE TOOL FOR THE DEVELOPMENT AND OUTCOMES OF GVHD.

Sabrina Berger da Silva[1], Tiago Paiva[1], Caroline de Mendonça da Silva[1], Luan Lopes Menezes[1], Glaucia Helena Martinho[1], José Aparecido Rezende[1], Gustavo Machado Teixeira[1], Vanessa Pinho da Silva[1], Robson Augusto Souza dos Santos[1], Mauro Martins Teixeira[1], Barbara Maximino Rezende[1]

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a key treatment for hematologic diseases such as leukemias, lymphomas, and sickle cell anemia. In Brazil, bone marrow is the most frequently used stem cell source due to accessibility. A major post-transplant complication is graft-versus-host disease (GVHD), occurring in 40–60% of recipients. GVHD results from immune imbalances caused by HLA mismatch, leading to donor T cell-mediated damage to organs like the skin, intestine, and liver. The renin-angiotensin-aldosterone system (RAAS) plays a regulatory role in inflammation. Among its components, angiotensin 1–7 [Ang-(1–7)]—produced by ACE2—is known for vasodilatory, anti-inflammatory, and anti-proliferative properties via Mas receptor activation. Previous studies, including those by our group, have shown that Ang-(1–7) attenuates inflammation in murine GVHD models, reducing both mortality and clinical severity.

OBJECTIVES:

To investigate whether blood levels of Ang-(1–7) are associated with the onset and severity of GVHD in patients undergoing allogeneic HSCT.

METHODS:

Patient recruitment is ongoing at a bone marrow transplant unit in Belo Horizonte. All eligible patients, regardless of age or sex, undergoing allogeneic HSCT are being included. A control group of healthy individuals matched by age and sex to transplanted patients will also be included. Blood samples are being collected at up to five time points: before conditioning (central catheter placement), on the day of transplantation, three days post-transplant, at GVHD diagnosis (if it occurs), and 20 days after diagnosis. Samples are processed to obtain plasma and buffy coat fractions, aliquoted, and stored at –80°C. Inflammatory mediators will be analyzed using two techniques: ELISA will quantify cytokines and chemokines IFN-γ, TNF-α, IL-6, IL-10, IL-17, CCL2, CCL3, and CCL5; mass spectrometry will measure angiotensin 1–7, angiotensin II, and alamandine.

RESULTS:

To date, 21 patients have been enrolled and are being clinically monitored up to 100 days post-transplant. Of these, 61.9% are female and 38.1% male. Regarding age, 52.4% are children (2–17 years), 33.3% adults, and 14.3% elderly. Diagnoses include acute myeloid leukemia (38.1%), sickle cell anemia (33.4%), myelofibrosis (4.8%), acute lymphoblastic leukemia (4.8%), beta-thalassemia major (4.8%), bone marrow aplasia (4.8%), and Chediak-Higashi syndrome (4.8%). Most patients (81%) received related donor transplants; 19% received unrelated transplants. Acute GVHD developed in 28.6% of patients, primarily among those with sickle cell anemia (50%), AML (33.3%), and Chediak-Higashi syndrome (16.7%).

CONCLUSION:

This study aims to establish Ang-(1–7) as a biomarker for predicting GVHD development and severity in HSCT patients. By integrating clinical follow-up and mediator profiling, we hope to improve early diagnosis and guide therapeutic strategies. Ethical approval: CAAE: 62353718.0.0000.5149

KEYWORDS: Angiotensin 1–7, Hematopoietic stem cell transplantation, Graft-versus-host disease

TARGETING GVHD INFLAMMATION: ANGIOTENSIN -(1–7) AS A PROTECTIVE ALLY IN BONE MARROW TRANSPLANTION

Caroline de Mendonça da Silva[1], Sabrina Berger da Silva[1], Luan Lopes Menezes[1], Tiago Paiva[1], Zara de Désirée Tonidandel Campos[1], Giselle Santos Magalhães[1], Marina Gomes Miranda e Castor Romero[1], Vanessa Pinho da Silva[1], Robson Augusto Souza dos Santos[1], Mauro Martins Teixeira[1], Barbara Maximino Rezende Gonçalves[1]

1. Federal University of Minas Gerais, Belo Horizonte – MG, Brazil.

INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a pivotal therapeutic strategy for various hematological diseases, including leukemia and lymphoma. However, graft-versus-host disease (GVHD) remains a common and severe complication, affecting 40–60% of recipients. GVHD occurs due to immunological mismatches in HLA molecules between donor and recipient, leading to immune activation and tissue damage in organs such as the liver, lungs, and intestines. Acute GVHD typically presents with skin rash, anorexia, nausea, diarrhea, weight loss, and mucosal lesions. The renin-angiotensin-aldosterone system (RAAS) plays a central role in cardiovascular and fluid homeostasis. One of its key bioactive peptides, angiotensin-(1–7) [Ang-(1–7)], is generated by ACE2 from angiotensin II and signals via the G protein-coupled Mas receptor. Beyond its vasodilatory and protective functions, Ang-(1–7) has demonstrated anti-inflammatory, anti-angiogenic, and anti-proliferative effects in diseases such as asthma, rheumatoid arthritis, and inflammatory bowel disease. Nevertheless, its role in GVHD pathogenesis remains unclear. Objectives: This study aimed to evaluate the effects of Ang-(1–7) on GVHD severity using a murine bone marrow transplant model.

METHODS:

Wild-type or Mas receptor-deficient (Mas KO) recipient mice were lethally irradiated and transplanted with 1 $\times 10^7$ bone marrow cells plus 3 $\times 10^7$ splenocytes from BALB/c donors. A treatment group received continuous subcutaneous infusion of Ang-(1–7) (1 µg/h) via osmotic pumps. Mice were monitored for survival and clinical GVHD scores. Liver histology and chemokine levels were assessed by ELISA, and blood smears were analyzed weekly. Data were analyzed using one-way ANOVA followed by the Newman-Keuls post hoc test. Results were considered statistically significant when $P < 0.05$ and expressed as mean ± SEM. Statistical analysis and graphing were performed with GraphPad Prism version 6. Results: Mas KO mice displayed greater liver damage, heightened inflammatory cell infiltration, increased neutrophil counts, and elevated CCL3/CCL5 levels, along with reduced survival and worsened GVHD clinical scores. Conversely, Ang-(1–7) treatment significantly decreased GVHD-associated mortality and morbidity.

CONCLUSION:

Mas receptor-deficient animals exhibit an exacerbated inflammatory response in the context of GVHD, leading to increased morbidity and mortality. In contrast, animals treated with Ang-(1–7) show reduced clinical scores and lethality, indicating that this molecule helps regulate GVHD-associated inflammation. Future Directions: We aim to further elucidate the mechanisms underlying Ang-(1–7)-mediated protection in GVHD and are currently conducting a prospective clinical study to investigate whether serum levels of angiotensin peptides correlate with GVHD severity in transplant patients. Ethical approval protocol: 4/2024

KEYWORDS: bone marrow transplantation, Graft-versus-host disease, Angiotensin 1–7

VAGUS NERVE STIMULATION AS AN INNOVATIVE THERAPEUTIC STRATEGY TO MODULATE INFLAMMATION AND IMPROVE OUTCOMES IN GRAFT-VERSUS-HOST DISEASE

Barbara Maximino Rezende[1], William Antônio Gonçalves[1], Luan Lopes Menezes[1], Caroline de Mendonça da Silva[1]; Sabrina Berger da Silva[1]; Tiago Paiva[1]; Vanessa Pinho[1], Mauro Martins Teixeira[1]

1. UFMG, Belo Horizonte - MG - Brasil.

INTRODUCTION:

Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is a curative therapy for hematologic diseases such as leukemia, lymphoma, and sickle cell anemia. However, graft-versus-host disease (GVHD) remains its most serious complication. It results from donor T cells attacking host tissues due to HLA mismatch, triggering immune activation, tissue damage, and release of cytokines like IFN-γ and TNF-α. Despite advances, GVHD continues to cause high morbidity and mortality. The cholinergic anti-inflammatory pathway (CAIP) is a neuroimmune mechanism through which the vagus nerve regulates inflammation via acetylcholine (ACh) and its receptors, especially α7 nicotinic ACh receptors. Vagus nerve stimulation (VNS), applied electrically or pharmacologically (e.g., with GTS-21), activates this pathway and has shown benefits in inflammatory diseases like rheumatoid arthritis. However, its role in GVHD remains poorly studied.

OBJECTIVE:

To investigate the role of CAIP in GVHD using electrical and pharmacological VNS.

METHODS:

C57BL/6J recipient mice underwent left cervical vagus nerve exposure and received a 2-minute electrical stimulation (5 Hz, 0.1 ms, 1 V). Six hours later, mice received total body irradiation (9 Gy, two doses) and were transplanted with 3×10^7 splenocytes and 1×10^7 bone marrow cells from Balb/c donors (GVHD+VNS group). The GVHD group received identical procedures without stimulation. The control group received syngeneic grafts (C57BL/6J to C57BL/6J). Clinical GVHD scores (0–12) were recorded every two days based on weight, piloerection, activity, posture, diarrhea, and fecal blood. Histopathology of jejunum, ileum, and liver, confocal intestinal imaging, and blood smears were performed. Cytokines and chemokines (IL-10, TNF-α, CCL2, CCL5) were measured in liver, intestine, and spleen by ELISA. Macrophage infiltration was assessed by NAG activity. Cardiac function was evaluated by echocardiography at days 14 and 35. A separate group received GTS-21 (4 mg/kg, i.p.) before irradiation.

RESULTS:

VNS reduced intestinal and hepatic damage, preserved spleen structure, and altered cytokine levels. IL-10, TNF-α, and CCL2 increased in the intestine, while TNF-α, CCL2, and CCL5 decreased in the liver. VNS also prevented leukopenia and neutrophilia, and reduced neutrophil and macrophage infiltration in spleen and liver. Cardiac output and ejection fraction were maintained, and interventricular septum thinning was prevented. Both VNS modalities reduced clinical signs and mortality in GVHD.

CONCLUSION:

Vagus nerve stimulation modulates inflammation and improves clinical outcomes in GVHD. These findings support its potential as an innovative therapeutic strategy in transplantation settings.

KEYWORDS: GVHD; Inflammation; Vagus Nerve

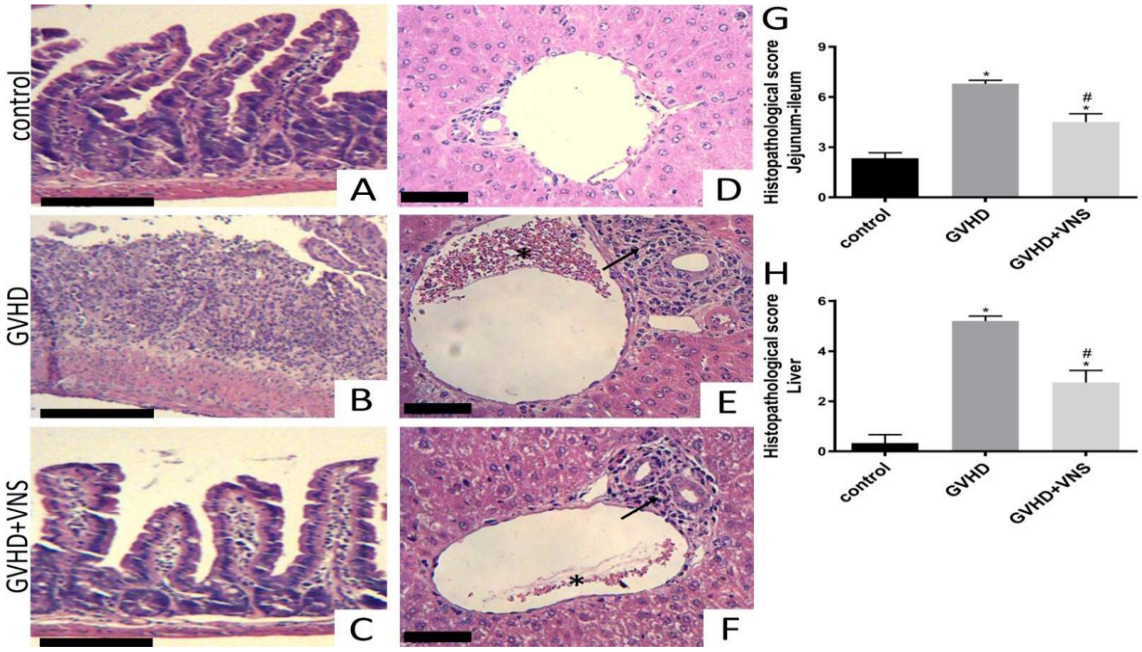


Figure 1. VNS reduces the intestinal and hepatic injury related to GVHD. Histopathological analysis of the intestine (A-C, G) and liver (D-F, H). Blades stained with H & E. Bar scale, 100 µm for the bowel panels and 50 µm for the liver panels. * and # P <0.05 compared to the control group and GVHD group, respectively.

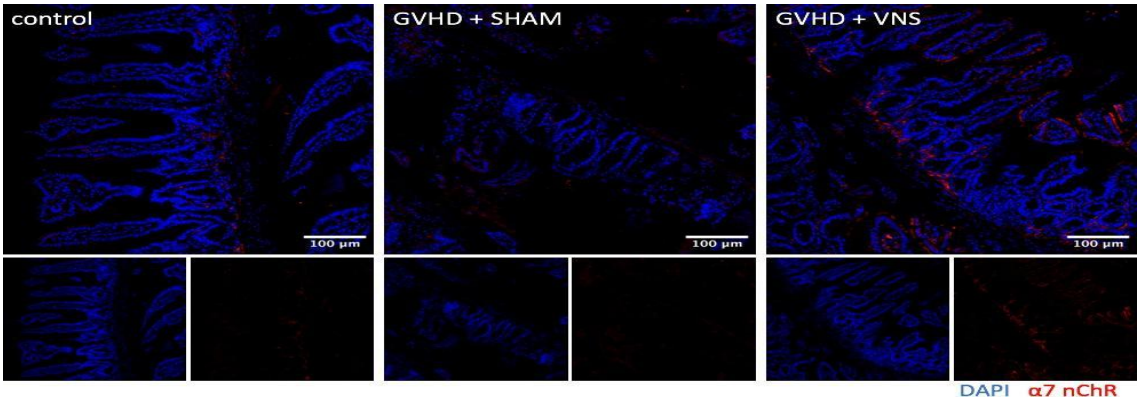


Figure 2. VNS prevents the expression of alpha-7 nicotinic receptor (α7nChR) in the jejunum-ileum of mice subjected to GVHD. Representative immunofluorescence images of the jejunum-ileum labeled for the alpha-7 nicotinic receptor (α7nChR). Blue: DAPI; Red: α7nChR.

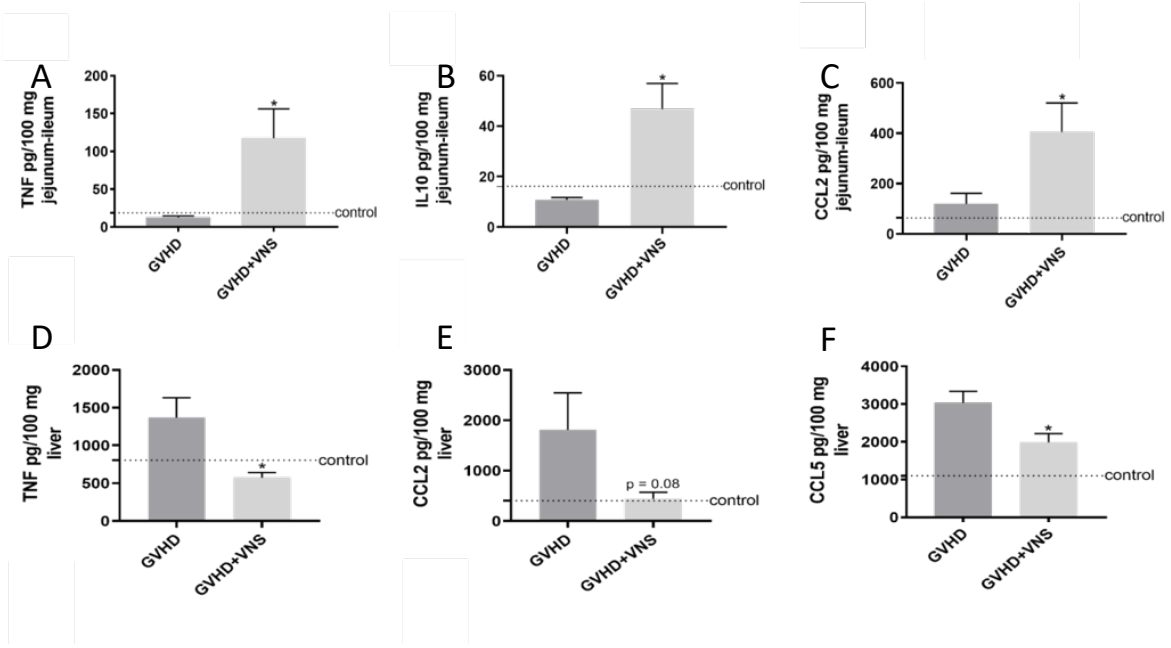


Figure 3. VNS increases TNF, IL10 and CCL2 in the jejunum ileum and reduces TNF, CCL2 and CCL5 in the liver of mice subjected to GVHD. Concentrations of TNF (A), IL-10 (B) and CCL2 (C) in the intestinal homogenates and TNF (D), CCL2 (E) and CCL5 (F) in the liver homogenates were evaluated by ELISA.

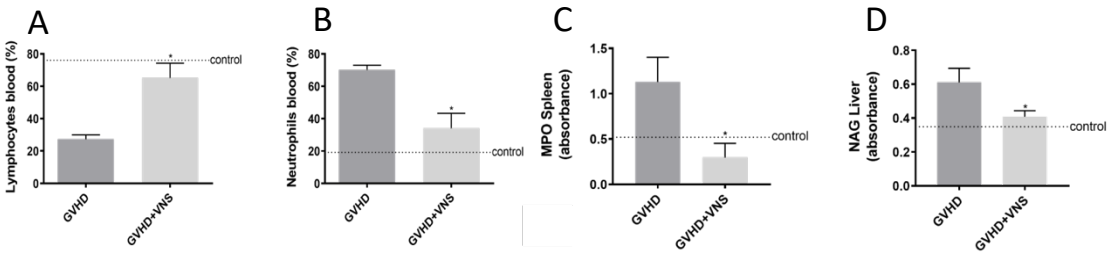


Figure 4. VNS prevents lymphopenia and neutrophilia related to GVHD and reduces neutrophils in the spleen and macrophages in the liver of mice subjected to GVHD. Percentage of lymphocytes (A) and neutrophils (B) in the blood. Concentrations of MPO (C) and NAG (D) were evaluated in homogenates from spleen and liver, respectively.

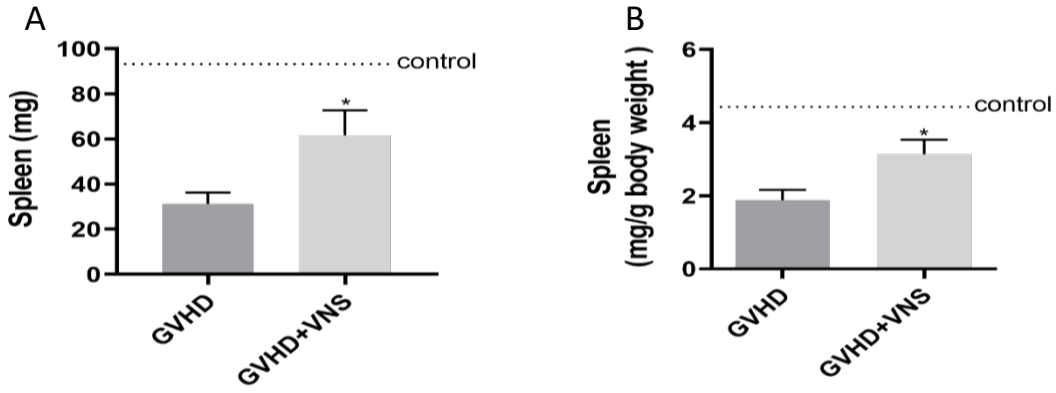


Figure 5. VNS inhibits splenic atrophy. The weight of the spleen was considered to evaluate atrophy (A) it was also evaluated in relation to the weight of the animal (B) respectively.

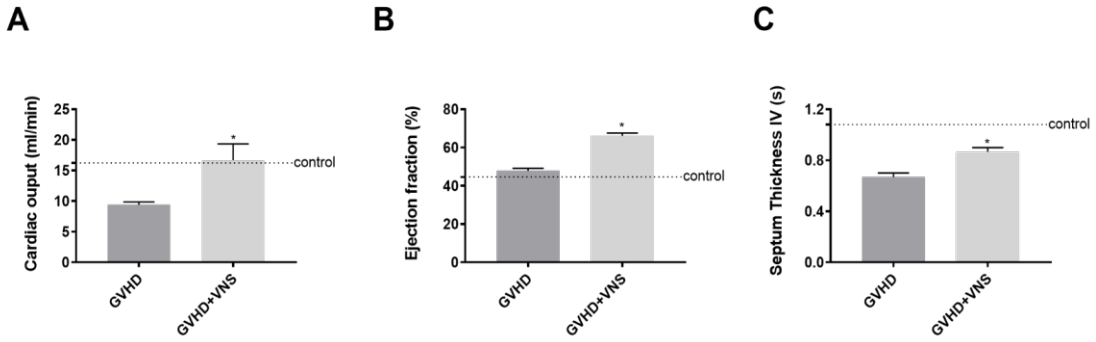


Figure 6. VNS prevented the cardiac damage related to GVHD . As also analyzed by echocardiogram the cardiac output (A) The Ejection Fraction (B) and The Septum Thickness (C), respectively

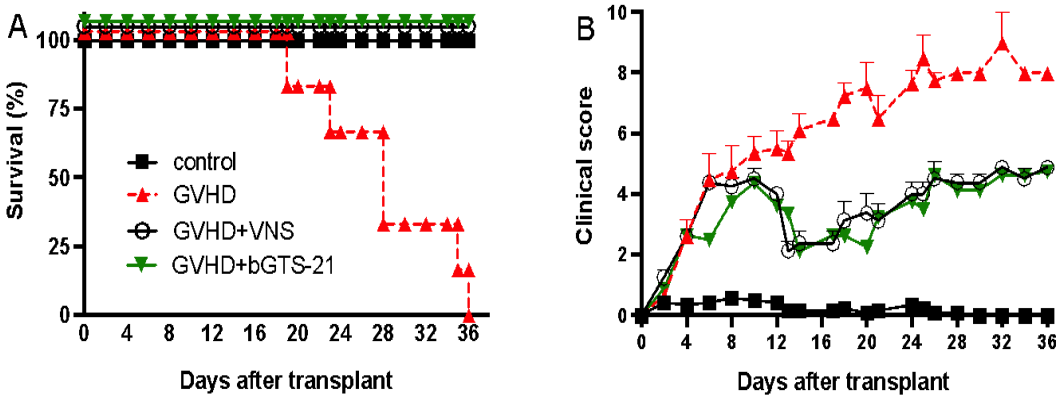
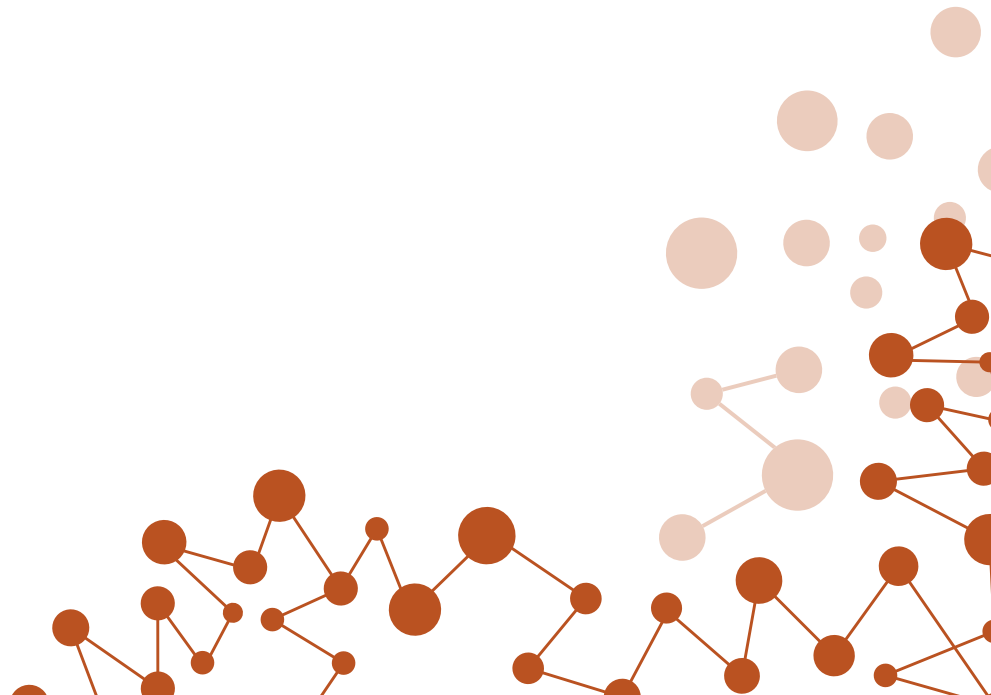


Figure 7. VNS AND GTS-21 prevents GVHD morbidity and mortality. After induction of GVHD, mice were monitored every 2 days for clinical score (A) and survival (B), respectively.

MULTIDISCIPLINARY

NURSING



ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FROM UMBILICAL CORD BLOOD IN PEDIATRIC PATIENTS: AN EXPERIENCE REPORT FROM THE NURSING PERSPECTIVE

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INTRODUCTION:

Umbilical cord blood transplants as a source of hematopoietic stem cells accounted for 1.5% of all hematopoietic stem cell transplants (HSCT) performed in Brazil between 2012 and 2021. HSCT involves multiple phases that require the involvement of a qualified multidisciplinary team, with nurses playing a central role due to their direct responsibility for patient care.

OBJECTIVES:

To describe the role of nurses in pediatric allogeneic HSCT using umbilical cord blood cells.

METHODS:

A descriptive report based on observational experience of three unrelated allogeneic HSCT procedures using umbilical cord blood-derived hematopoietic stem cells, carried out in 2025 at a reference hospital for pediatric onco-hematology in southern Brazil.

RESULTS:

The diagnoses of the observed cases were: B-cell acute lymphoblastic leukemia, bone marrow aplasia, and Diamond-Blackfan anemia. In two cases, the conditioning regimen used was BuFluCy (busulfan, fludarabine, and cyclophosphamide), and in one case, FluCyTBI (fludarabine, cyclophosphamide, and total body irradiation). Nursing care during chemotherapy administration included double-checking the patient's identification and prescribed dosage at the bedside by two nursing team members, with the legal guardian present, immediately before infusion. For patients receiving busulfan, blood samples were collected for serum level monitoring immediately after infusion and again one, two, and four hours post-infusion. Nurses are responsible for managing infusion schedules and sequences, ensuring timely sample collection for accurate pharmacokinetic assessment. The nursing team also checks all materials required for HSCT, monitors and assesses vital signs at the beginning of and every 10 minutes during the infusion, and every hour thereafter. The stem cell infusion itself is performed by the nurse. Throughout the entire process, central venous catheter assessments, sample collections, and dressing changes are performed exclusively by nurses. The entire nursing team is involved in catheter-related care during intravenous drug administration, including monitoring for drug interactions and using appropriate techniques for catheter flushing—such as the push-pause and positive pressure methods—to maintain catheter integrity, support procedural success, and prevent patient harm.

CONCLUSION:

The nursing team's role spans all phases of HSCT, proving essential to the successful infusion of hematopoietic stem cells and ensuring patient safety throughout the entire process.

KEYWORDS: Nursing Care; Nurses, Pediatric; Oncology Nursing;

ANALYSIS OF REFERRAL TIME FOR BONE MARROW TRANSPLANTATION IN THE SUS: THE IMPACT OF PROADI-SUS ON BMT PROCEDURES

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Bone Marrow Transplantation (BMT) is essential for treating severe hematologic diseases such as leukemia and anemia, enabling the replacement of diseased stem cells with healthy ones. In Brazil, limited hospital infrastructure, long waiting times, and high costs hinder access. However, the Institutional Development Support Program of the Unified Health System (PROADI-SUS) has helped expand access by training professionals and optimizing processes. Partnering with top hospitals, it has reduced disparities and improved patient outcomes.This study aimed to analyze referral time, hospitalization, and BMT execution for patients referred via the National Transplant System (SNT), based on demographic and clinical data. A cross-sectional study was conducted using data from male and female patients undergoing BMT for benign or malignant diseases through PROADI-SUS (June/2022 to May/2025). Cases were regulated by the SNT.Variables included demographics, clinical data, and time intervals (in days) between referral, hospitalization, and BMT, and BMT types (related, autologous, haploidentical, unrelated). Descriptive analysis was performed. The Mann-Whitney test compared two groups; Kruskal-Wallis and Dunn's tests were used for three or more. Significance was set at $p<0.05$. Data were collected via REDCap.A total of 82 patients were analyzed; 53.7% were female. Mean age was 35.1 years (SD=19.6), median 35 (range: 4–70). Median age was 10 for children/adolescents and 45 for adults. Haploidentical transplants were most common (47.6%, n=39), followed by related (29.3%, n=24). Acute myeloid leukemia was the most frequent diagnosis (26.8%, n=22), followed by acute lymphoblastic leukemia (14.6%, n=12). Eight patients had benign diseases. Median times were: SNT to hospitalization – 89 days [9–294]; hospitalization to BMT – 7 days [1–60]; total time SNT to BMT – 97.5 days [48–301]. About 60% had clinical complications, with a significant difference between groups ($p<0.010$). For patients without complications, medians were 65, 6, and 71 days respectively.There were no significant differences by state of residence. However, time from hospitalization to BMT varied by transplant type ($p<0.001$). Autologous BMT had shorter time than haploidentical (–3 days; $p<0.001$); related vs. haploidentical also differed (–2 days; $p=0.011$).In conclusion, delays were mainly due to complications, delays in pre-BMT exams, and pre-treatment needs. Despite longer times for complicated cases, the data suggest PROADI-SUS improved access and reduced disparities. The high rate of haploidentical BMTs reflects new strategies amid donor scarcity. PROADI-SUS plays a vital role, and continued investment in reducing delays and preventing complications is crucial.

KEYWORDS: BMT, SUS, PROADI

APPLICATION OF CLINICAL DETERIORATION SCORE TO SUPPORT DECISION-MAKING IN THE CARE OF PATIENTS UNDERGOING BONE MARROW TRANSPLANTATION

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INTRODUCTION:

The bone marrow transplant (BMT) process can present serious complications and increase the risk of mortality, requiring the transfer of the patient to an intensive care unit (ICU). Continuous and early monitoring of clinical deterioration is essential for rapid and effective intervention. The National Early Warning Score (NEWS) is a tool used for the early identification of signs of clinical deterioration. It provides an objective and systematic approach that assists in the escalation of care, decision-making: such as intensive monitoring and ICU transfer. Additionally, it promotes clear communication among healthcare teams when describing the patient's condition and the urgency of interventions, allowing for a rapid, targeted response and contributing to better clinical outcomes and a reduction in complications.

OBJECTIVES:

To report on the monitoring conducted by a Care Monitoring Operations Center (CMOA), using the NEWS score during the care of BMT patients.

METHOD:

This is a report of experience from a hematology and BMT inpatient unit of a large hospital in São Paulo.

RESULTS:

A pilot project was developed to monitor patients during the BMT process in collaboration with the CMOA, supported by an intensivist physician, to enable interventions based on the NEWS score. Decision-making is discussed with the medical and multidisciplinary care team and facilitates the escalation of care, such as: the need for more frequent vital signs checks, bedside monitoring, activation of the rapid response team (RRT), if necessary, ICU transfer. The project, which is currently ongoing, monitored around 250 onco-hematologic patients from January to May, including BMT patients.

CONCLUSION:

Throughout the monitoring process, improved outcomes and early interventions were observed. Additionally, a significant cultural shift was noted, valuing tools like the NEWS score and metric data analysis to support better decision-making for interventions and treatments. In this way, unnecessary interventions can be reduced, and a safer environment with higher quality of care can be promoted.

CASE REPORT: CENTRAL VENOUS CATHETER USE IN A HEMATOPOIETIC STEM CELL TRANSPLANT PATIENT WITH MULTIPLE VENOUS OCCLUSIONS

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INTRODUCTION:

Hematopoietic stem cell transplants (HSCT) require long-term venous access, with Hickman® catheters being the most commonly used. However, thrombosis sequelae from prior treatment are observed in this population, potentially impacting HSCT viability.

OBJECTIVE:

To describe the case of a patient with occlusion of all upper-body central veins indicated for HSCT.

METHOD:

This is a case report. Data were collected through a review of the electronic medical record (CAAE Nº 81745718100005327).

RESULTS:

RS, a 45- years-old male, was referred for haploidentical HSCT due to anaplastic large cell T-lymphoma in first remission after extensive treatment. Prior to HSCT, occlusion of the superior vena cava (SVC), brachiocephalic veins, subclavian, axillary, and internal jugular veins was identified due to previous thrombosis. A femoral inserted central catheter (FICC) was chosen, 5F triple-lumen, inserted into the left femoral vein, tunneled and externalized in the anterior medial thigh (vastus medialis muscle). Ultrasound-guided puncture and fluoroscopy were used. The tunnel measured 13 cm, with a catheter-to-vessel ratio of 5%. Internal length was 55 cm, and the distal end of the FICC was positioned in the inferior vena cava. The patient underwent conditioning with FLU/CY/TBI protocol. Fresh stem cells were infused and neutrophil engraftment occurred on Day +23. During this period, several complications arose: febrile neutropenia, hemorrhagic cystitis, urinary infection, SVC syndrome, and cytomegalovirus (CMV) reactivation. On Day +48, hyperemia, warmth, and pain were observed in the FICC subcutaneous tunnel, suggesting cellulitis. Blood cultures identified Enterococcus faecalis, and the catheter was replaced after 56 days. The new FICC, 5F double-lumen, was inserted into the right femoral vein and externalized in the anterior medial thigh, with a 10 cm tunnel. Multiple post-HSCT complications occurred: grade IV hemorrhagic cystitis due to BK and JC viruses, orchiepididymitis, urinary infection, CMV reactivations, prostatitis, aspergillosis, acute kidney injury from immunosuppressants, chronic graft-versus-host disease with severe gastrointestinal involvement, and thrombotic microangiopathy. Two blood cultures isolated Pseudomonas aeruginosa from urinary focus, with clinical response to treatment. The patient developed progressive hypoxemia and died on Day +206. The second catheter remained for 155 days.

CONCLUSION:

Peripheral central catheters are less commonly used in HSCT due to the assumption that prolonged infusion through smaller-caliber catheters may impair cell viability. However, some studies have shown this does not affect HSCT outcomes. In this case, the tunneled FICC enabled HSCT. Although this location is associated with higher rates of infection and/or sepsis, tunneling technique allowed better positioning, reducing risks, and supported outpatient follow-up in the post-transplant.

KEYWORDS:

Central Venous Catheters, Hematopoietic Stem Cell Transplantation, Advanced Nursing Practice.

DESIGN AND IMPLEMENTATION OF AN EDUCATIONAL BOOKLET FOR PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Hematopoietic Stem Cell Transplantation (HSCT) is a highly complex therapeutic procedure indicated for various hematological, oncological and autoimmune diseases[1]. It is divided into seven stages of care, beginning with the pre-hospitalization outpatient stage up to the late outpatient stage after day +100[2]. During these stages, multiple pieces of information are transmitted to the patient and their support network by a multidisciplinary team, in a short time, which can impact treatment adherence and clinical outcomes. Faced with this challenge, transplant nurses and doctors identified the need for an educational tool offering written guidelines to facilitate understanding and organization of this information, promoting safety, autonomy and co-responsibility in patient care during HSCT stages.

OBJECTIVE:

To report the experience of transplant nurses and doctors in designing and implementing a patient booklet as an educational strategy that gathers the main information related to HSCT care.

METHOD:

This is an experience report from professionals involved in creating and implementing the HSCT patient booklet at a 100% SUS Transplant Center in Minas Gerais, Brazil. RESULTS: The booklet's development was guided by common questions, difficulties, and demands observed during patient follow-up. Three stages were considered: the pre-HSCT outpatient phase, the hospitalization (transplant) phase, and the post-HSCT outpatient follow-up[3]. The content was organized into sections with general information on HSCT and practical guidance provided by the multiprofessional team. The language was accessible, and visual resources were included to support understanding. The material was reviewed, adjusted, and validated by the transplant center's team. It also contains fields for manual entries, such as catheter removal, dressings, and appointments, promoting integrated and systematized monitoring of care. Booklet distribution began in April 2025.

CONCLUSION:

The HSCT patient booklet proved to be a promising educational strategy by facilitating the organization, systematization, and continuous access to guidance related to the transplant process. This tool strengthens patient-centered care by encouraging autonomy, safety, and co-responsibility. Future assessments of patient adherence to the booklet and validation by HSCT specialists are recommended to ensure its effectiveness and applicability.

KEYWORDS: Hematopoietic Stem Cell Transplantation; Health Education; Health Communication

DEVELOPMENT OF A COURSE FOR TRAINING NURSES IN HEMATOPOIETIC STEM CELL TRANSPLANTATION ON THE EXPERT PATIENT PROGRAM

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Estéfany Alves Augusto[1], Edmundo João Mansilla Cordeiro[1], Adriana Catarina de Souza Oliveira[3]
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5 Department of Clinical Medicine; Federal University of Rio Grande do Norte, Natal, Brazil.

INTRODUCTION:

Care for patients undergoing Hematopoietic Stem Cell Transplantation (HSCT) requires a focused and transformative approach that promotes empowerment, autonomy, and quality of life in the therapeutic process. The use of educational technologies can favor the instrumentalization, acquisition of knowledge, and empowerment of patients, being an efficient strategy for disease control and treatments. Within the multidisciplinary team, the nurse is responsible for caring for, managing, teaching, and researching viable solutions to provide changes in the patient's autonomy and quality of life.

OBJECTIVE:

To develop an educational technology for the training of hematopoietic stem cell transplant nurses on the Expert Patient Program.

METHOD:

This is a methodological study based on the psychometric framework of Pasquali et al. (2010) and developed from the Instructional System Design model known by the acronym ADDIE, which has five phases, namely: analysis, design, development, implementation, and evaluation. It is worth mentioning that this research concluded up to the development phase of the ADDIE model, which refers to the structuring of educational technology. The course was validated by 18 expert judges in the area of HSCT in Brazil. The study was approved by the Ethics and Research Committee, under CAAE No. 55774621.5.0000.5537.

RESULTS:

Through a situational diagnosis, the need for educational intervention on the Expert Patient Program within the scope of HSCT was observed. Then, the learning objectives, contents and pedagogical structure of the course were defined. To support this phase of the research, a scope review and a focus group with nurses working in an HSCT service were carried out in order to concatenate the contents necessary for training nurses on the Expert Patient Program. The course was organized into five moments/classes with a schedule of diverse activities. Finally, through the evaluation of the expert judges on the content and appearance of the course, it was possible to demonstrate that it is valid in its appearance (88%) and content (87%).

CONCLUSION:

The results demonstrated the possibility of HSCT being a center of transformation by integrating education, autonomy and expanded care through the development of the course for training HSCT nurses on the Expert Patient Program with a view to becoming capable educators with the knowledge and skills necessary to act in the recognition, recruitment and training of leading, empowered and protagonist patients who can promote transformation in the context of HSCT.

KEYWORDS:

Nursing; Educational Technology; Hematopoietic Stem Cell Transplantation.

FUNDERS:

CNPq with master's scholarship.

DEVELOPMENT OF A DATA COLLECTION INSTRUMENT FOR SITUATIONAL DIAGNOSIS AND MONITORING OF IMPROVEMENT PLANS IN THE CARE AND THERAPEUTIC PROCESS OF HEMATOPOIETIC STEM CELL TRANSPLANTATION IN HOSPITALS WITHIN THE UNIFIED HEALTH SYSTEM IN BRAZIL

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1. BP – A Beneficência Portuguesa de São Paulo

INTRODUCTION:

As part of a project by the Institutional Development Support Program of the Unified Health System in partnership with a healthcare institution in São Paulo, visits are being conducted to hematopoietic stem cell transplant (HSCT) centers across different regions of Brazil. The goal is to obtain a situational diagnosis, identify opportunities for management and process improvements, and create specific action plans for each visited center, with ongoing follow-up. This approach aims to ensure more equitable access for patients undergoing HSCT. The development of a single data collection instrument during these visits seeks to standardize the evaluation process and facilitate the implementation of improvements.

OBJECTIVE:

To describe the process of developing a data collection instrument for conducting diagnostic and follow-up visits, ensuring uniform mapping across centers.

METHODOLOGY:

A descriptive study. Based on current legislation, quality manuals, and best available clinical practices, a team of physicians and nurses developed an instrument using the REDCap research data platform.

RESULTS:

The instrument is organized into two parts: Center Identification and Assessment by blocks: divided into 12 blocks. Block 1: Management and leadership in HSCT (7 questions focused on process mapping and indicators). Block 2: Quality, patient safety and education (17 questions about quality processes and specialized training). Block 3: Multidisciplinary team (30 questions about the different professional roles involved and their activities pre, intra, and post-transplant). Block 4: Medical team (22 questions related to regulation, transplant indication, structured evolution, pre-transplant exams, and donor evaluation). Block 5: Pre-HSCT (18 questions about assessment and effective communication). Block 6: Inpatient unit (17 questions about physical structure, air quality control, water safety, and healthcare risks). Block 7: Medical record documentation (35 questions about unifying important information). Block 8: Support areas (13 questions about relationships with emergency services, intensive care units, laboratories, and support houses). Block 9: Hemotherapy (53 questions evaluating the safety and quality of the transfusion service). Block 10: Cell therapy (69 questions related to storage and infusion of hematopoietic stem cells). Block 11: Donor (4 questions). Block 12: Post-HSCT (7 questions about follow-up and monitoring to ensure continuity of care).

CONCLUSION:

The development of the real-time filling instrument enabled more accurate and efficient data collection by unifying information about visits. Technical reports are generated uniformly, facilitating the creation of individualized action plans. The standardization of transplant centers helps promote continuous improvements and ensures that patients receive safe, high-quality care throughout the entire HSCT process, increasing the chances of success and patient survival.

KEYWORDS:

Hematopoietic stem cell transplant, Unified Health System, data collection instrument

DEVELOPMENT OF INSTRUCTIONAL MATERIAL FOR NURSING IN HEMATOPOIETIC CELL TRANSPLANTATION: A STRATEGY FOR PROFESSIONAL TRAINING

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INTRODUCTION:

Hematopoietic Cell Transplantation (HCT) is a highly complex procedure requiring specialized nursing care. As transplant centers expand across Brazil, forming adequately trained nursing teams has become an increasing challenge. This gap appears to stem from the limited inclusion of HCT-related content in undergraduate and postgraduate nursing curricula, as well as the absence of residency or specialization programs focused exclusively on HCT. Therefore, there is an urgent need to provide instructional materials that address the specificities of nursing care in HCT.

OBJECTIVE:

To develop instructional educational material to enhance nursing competencies in the care of patients undergoing hematopoietic stem cell transplantation, based on the best available evidence and specialized clinical practice.

METHOD:

This is a methodological study carried out between December 2024 and June 2025, structured in two phases and nine stages. The target audience comprises nurses working in HCT transplant centers. The content was developed by experienced professionals in the field, with contributions from experts in cell processing, graphic design, and medical photography. The choice of a printed book format responded to an expressed demand from nurses working in various care settings.

RESULTS:

The final product was a printed book comprising seven updated chapters, aligned with national and international guidelines, covering essential aspects of HCT nursing care. The chapters address: 1) Basic Principles of Hematopoietic Cell Transplantation: Biological Foundations and Therapeutic Modalities; 2) Sources and Collection of Hematopoietic Cells: Procedures for Bone Marrow, Peripheral Blood, and Umbilical Cord and Placental Blood Collection; 3) Pre-Transplant Phase: From Indication to Conditioning Regimen; 4) Intra-Transplant Phase: From Day Zero to Engraftment; 5) Post-Transplant Phase: From Hospital Discharge to Outpatient Follow-Up; 6) CAR-T Cell Therapy: Basic Principles and the Nurse's Role; 7) Hematopoietic Cell Transplantation Unit: Physical Structure and Human Resources for Implementing and Operating Transplant Services.

CONCLUSION:

The developed material aims to support nursing practice by promoting patient safety, delivering comprehensive care, and reinforcing the autonomous and scientific role of nurses in the care of immunosuppressed patients. This work represents a concrete strategy to support continuing education and the technical-scientific advancement of nursing in one of the most complex areas of clinical practice.

KEYWORDS:

Hematopoietic Stem Cell Transplantation, Evidence-Based Nursing, Professional Training.

EXPANSION IN TRANSPLANT VOLUME FOLLOWING THE ENHANCEMENT OF NURSING NAVIGATION IN THE PRE-BONE MARROW TRANSPLANT OUTPATIENT CLINIC OF A LARGE-SCALE HOSPITAL

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INTRODUCTION:

Bone Marrow Transplantation (BMT) involves several stages and requires a qualified multidisciplinary team. Nurses play a key role in this process, acting from the pre-transplant phase through to the management of post-transplant complications.[1] Considering the complexity of the treatment, patient navigation has emerged as a support tool and a distinguishing factor in BMT services. The nurse navigator contributes both during the pre-transplant phase and in overcoming barriers to accessing the healthcare system.[2]

OBJECTIVE:

To present the results of the growth of the BMT service following the expansion of nursing navigation in the outpatient clinic, highlighting its impact on the quality of care and transplant outcomes.

METHODOLOGY:

A comparative analysis was carried out between 2023 and 2024, considering the volume of transplants performed. In 2023, there was one nurse navigator for allogeneic cases and another nurse working in the outpatient clinic who also handled autologous patients, complications, post-transplant care, and other sector demands. This setup limited the ability to provide individualized navigation.

In 2024, the team was restructured with the addition of a dedicated nurse exclusively responsible for navigating autologous transplant cases, working full-time from Monday to Friday. This change was based on a first-quarter assessment of the number of initial consultation requests and the internal waiting list for inpatient admissions. A follow-up model was then developed with a clear division of responsibilities between the allogeneic and autologous nurse navigators. This allowed for a more personalized approach, greater patient support, and improved service organization.

RESULTS:

In 2023, a total of 107 bone marrow transplants were performed, including 47 allogeneic and 60 autologous procedures. In 2024, with the expansion of nursing navigation, the total increased to 150 transplants: 67 allogeneic (a 42.55% increase) and 82 autologous (a 36.67% increase). This marked the highest number since the service was established, reflecting the direct impact of the new navigation structure. In addition to this growth, patient follow-up became more efficient and humanized, improving care management, hospitalization scheduling, and overall patient support.

CONCLUSION:

The expansion of nursing navigation was essential for the growth of transplants in 2024. With increases of 42.55% in allogeneic and 36.67% in autologous transplants, the service began offering more specialized, efficient, and humanized care, positively impacting clinical outcomes. This model can serve as a reference for other institutions aiming to enhance patient care and expand their BMT services.

KEYWORDS:

Navigation, Bone Marrow Transplantation, Transplant Outpatient Clinic

FIVE YEARS OF BONE MARROW TRANSPLANTATION AT THE CHILDREN'S HOSPITAL OF BRASÍLIA: EPIDEMIOLOGICAL OVERVIEW AND CLINICAL OUTCOMES IN PEDIATRIC PATIENTS

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INTRODUCTION:

Bone marrow transplantation (BMT) has transformed the treatment of pediatric diseases, improving survival rates and quality of life. At the Bone Marrow Transplantation Unit of the Hospital da Criança de Brasília José Alencar (HCB), the service was established over the last five years: autologous transplants began in 2019, related allogeneic transplants in 2022, and unrelated allogeneic transplants in 2024.

OBJECTIVE:

To analyze epidemiological and clinical indicators of the BMT Unit at HCB between September 2019 and September 2024.

METHODS:

A descriptive, retrospective study including all patients who underwent any type of BMT. Data were collected from the unit's management database. Variables included sex, age, state of origin, diagnosis, transplant modality, conditioning regimen, mean time to engraftment, overall survival, and graft failure.

RESULTS:

A total of 80 autologous, 39 related allogeneic, and 5 unrelated allogeneic transplants were performed. The mean age was 7 years. In allogeneic transplants, 72% of patients were from the Central-West region, predominantly the Federal District (43%). In autologous transplants, the Central-West region accounted for 65%. Notable cases included two Indigenous patients (5%) and one migrant (2%). Primary autologous diagnoses: Neuroblastoma (45%), Hodgkin's Lymphoma (17%), and Germ Cell Tumors (14%). In related allogeneic transplants: Sickle Cell Anemia, B-Cell Acute Lymphoblastic Leukemia, and Bone Marrow Aplasia (23% each). In unrelated allogeneic transplants: Fanconi Anemia (40%), Hemophagocytic Lymphohistiocytosis, Osteopetrosis, and Chronic Granulomatous Disease (20%). Mean engraftment time was 17 days (related allogeneic), 18 days (unrelated allogeneic), and 14 days (autologous). Graft failure occurred in 3 patients (6.3%). Overall survival for autologous transplants was 78% at 1 year and 40% at 5 years; for allogeneic transplants, it was 80% at 1 year and 78% at 2 years.

CONCLUSION:

The study demonstrates the consolidation of the HCB BMT Unit as a regional reference, with strong clinical outcomes and expanded transplant modalities, underscoring its importance in specialized pediatric care.

KEYWORDS:

Bone Marrow Transplantation, Pediatrics, Health Services Epidemiology.

FLOWCHART FOR NURSING CARE IN OUTPATIENT AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

The flowchart consists of a graphical and algorithmic representation tool of a process, in a logical and simplified sequence. Thus, this technology is essential to qualify the care provided by nursing, especially those of greater complexity, such as outpatient autologous hematopoietic stem cell transplantation, as it provides greater safety and efficiency in decision-making during care for patients undergoing this treatment. However, despite the importance of these instruments, no management technology applied to this context has been verified in the scientific literature.

OBJECTIVE:

To develop a flowchart for nursing care for patients undergoing outpatient autologous hematopoietic stem cell transplantation.

METHODS:

This is a methodological study with a quantitative approach, guided by Pasquali's psychometric framework, structured in theoretical procedures, with the performance of a Scoping Review and a focus group with the nursing team of a reference hospital for this type of procedure in the state of Rio Grande do Norte, Brazil. In the empirical pole, the flowchart was constructed and the content and appearance of the tool were validated by nurses who are specialists in the area, using the Delphi technique, with the evaluation of the criteria proposed by Pasquali et al. (2010) and the Suitability Assessment of Materials. In the analytical pole, the content validation coefficient and inter-judge agreement were considered. The study was approved by the Research Ethics Committee of the Federal University of Rio Grande do Norte under CAAE: no. 58796722.8.0000.5537.

RESULTS:

Initially, the study mapped the nursing care described in the scientific literature and by the participants of the focus group of the nursing team that included: the Pre-transplant stage, with the performance of chemotherapy conditioning, mobilization and collection of hematopoietic stem cells; on Day Zero, with thawing of the bag and reinfusion of hematopoietic stem cells; and, in the Post-transplant period, which described the main guidelines for discharge, home care and medication use. After developing the flowchart, it was sent to the judges for the first Delphi round and achieved a total content validation coefficient > 0.9. After the proposed adjustments, in the second round the content and appearance validity indexes reached values > 0.9 and 90% agreement.

CONCLUSION:

The results showed that the proposed method was effective for developing and validating the content and appearance of a flowchart to organize nursing care in outpatient services for autologous hematopoietic stem cell transplantation in a standardized, efficient, assertive and standardized manner.

KEYWORDS: Nursing Care, Workflow, Hematopoietic Stem Cell Transplantation.

FROM CHALLENGE TO EXCELLENCE: STRATEGIES FOR IMPLEMENTING A BONE MARROW TRANSPLANT PROGRAM

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INTRODUCTION:

Implementing a bone marrow transplant (BMT) program is a key strategy for expanding access to treatment for severe hematologic diseases like leukemias, lymphomas, and multiple myeloma conditions for which transplantation may be the only curative option. The BMT unit is structured to serve adult patients and features a semi-intensive care model with 12 individual rooms equipped with HEPA filters, along with all the necessary infrastructure and protocols to maintain a protective environment essential for the safety and success of the treatment. Besides documentation and regulatory compliance, the implementation process included comprehensive training for nursing and multidisciplinary teams to ensure competence aligned with the specific needs of this patient population. Objective: To assess the challenges, initial outcomes, and future perspectives associated with the implementation of a BMT program in a large philanthropic hospital.

METHOD:

This is a descriptive observational study with prospective data collection concerning the implementation process. The process began through the development of a comprehensive action plan involving all hospital departments, supported by monthly coordination meetings. A total of 40 training sessions utilizing active learning methodologies were conducted for the multidisciplinary team. Legal and technical documentation (protocols and standard operating procedures) was created, and the physical infrastructure was adapted to meet the required standards.

RESULTS:

Key challenges identified included delays in completing the physical infrastructure certified in September 2023 and finalized in July 2024 with the official opening of the unit the recruitment and training of a multidisciplinary team, and the implementation of care protocols, which was initially hindered by team fragmentation. During the first eight months of operation (August 2024 to April 2025), 17 autologous transplants were performed for patients diagnosed with lymphomas and myelomas. The engraftment rate was 100%, with a mean time to engraftment of 11 days.

CONCLUSION:

Despite initial challenges, the implementation of the BMT program proved to be both feasible and safe, with clinical outcomes surpassing national and international benchmarks. The program expanded regional access to advanced therapies and reduced the need for patient travel to distant centers. Strengthening the team, standardizing processes, and continuing investments in training are essential for the program's sustainability and future expansion, with expectations of increasing procedure volume and incorporating new transplant modalities.

KEYWORDS: Health Management, Bone Marrow Transplantation, Healthcare Quality

IMMUNIZATION OF POST-HEMATOPOIETIC STEM CELL TRANSPLANT PATIENTS: A LITERATURE REVIEW

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INTRODUCTION:

Vaccines are a form of immunity promotion that demonstrate effective results in disease prevention. They are procedures that mimic immunity and utilize products containing attenuated microorganisms, inactivated agents, or parts thereof. Allowing the organism to contact a component of the parasite enables the development of mechanisms to combat invaders and to establish immunological memory in case of future exposure to the pathogen. Patients post-hematopoietic stem cell transplantation (HSCT) require vaccination schedules to develop immunity, as, after transplantation, T and B lymphocytes and immunoglobulins are below normal levels. However, this group often feels apprehensive about resuming routine activities, and one common concern is regarding the use of immunizations. The need for robust scientific knowledge and evidence-based practice motivated this research.

OBJECTIVE:

To review and synthesize the main findings on this topic.

METHODS:

A literature review was conducted with the guiding question: what evidence does the literature provide regarding the importance of immunizations in post-HSCT patients? The search was performed across the LILACS, MEDLINE, SCIELO, and BDENF databases from 2014 to 2024. Descriptors and Boolean operators used were: "hematopoietic stem cell transplantation" AND "reimmunization" AND "healthcare" AND "vaccination." Inclusion criteria encompassed full articles and dissertations in Portuguese, published and available in full text. Exclusion criteria included theses, proceedings, and editorials.

RESULTS:

Sixteen studies were identified, of which eleven met the inclusion criteria. Post-transplant vaccination recommendations are the same for autologous, allogeneic, or syngeneic transplant recipients. Immunological reconstitution is essential for transplant success, as it plays a vital role in defending against pathogenic agents. The process resembles immune development during early embryonic life. The high morbidity and mortality rates among post-transplant patients are related to delayed immune system recovery. The revaccination program is complex, involving 12 to 14 different vaccines, multiple doses, varying intervals, and restrictions on live attenuated vaccines. Nurses, who provide continuous care in HSCT, are among the primary professionals responsible for vaccination and are considered natural educators within the healthcare context, capable of clarifying doubts and providing guidance.

CONCLUSION:

Due to its high complexity, the post-HSCT period involves a prolonged recovery marked by uncertainties and fears. This situation may discourage patient adherence to the vaccination schedule, highlighting the need for healthcare professional guidance. The systematic review identified a gap in the existing literature, with few studies meeting the inclusion criteria. Further research is necessary to generate more robust evidence.

KEYWORDS: Hematopoietic stem cell transplantation. Reimmunization. Nursing.

IMPLEMENTATION OF A BUSINESS INTELLIGENCE (BI) TOOL FOR OPTIMIZATION OF CLINICAL AND OPERATIONAL MANAGEMENT IN HEMATOPOIETIC CELL TRANSPLANTATION (HCT) AND CELLULAR THERAPY PROGRAMS

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INTRODUCTION:

Hematopoietic Cell Transplantation (HCT) is an essential therapeutic strategy for the treatment of severe hematologic diseases. The complexity of care and the growing demand for quality, safety, and efficiency in HCT programs and other types of cellular therapies pose significant challenges to clinical and operational management. The increasing digitalization of electronic medical records and the accumulation of large volumes of clinical, laboratory, and administrative data require technological solutions that support rapid, evidence-based decision-making. The structured use of Business Intelligence (BI) tools enables real-time data integration, visualization, and interpretation, supporting decision-making, complication monitoring, and the clinical and economic performance evaluation of HCT programs.

OBJECTIVE:

To develop and implement a BI model aimed at monitoring clinical, care-related, and operational indicators in an HCT and Cellular Therapy program certified by FACT, with the goal of improving management efficiency, patient safety, and clinical outcomes.

METHOD:

This is a study focused on the development and implementation of health technology, using a quantitative and descriptive approach. Retrospective and prospective data will be used from patients undergoing HCT (autologous and allogeneic) in a reference HCT center within the Brazilian private healthcare sector. The methodology includes the following steps: (1) Process mapping of the program and definition of key performance indicators (KPIs), including clinical (time to engraftment, infection incidence, 100-day survival), and operational indicators (program coverage area, target population); (2) Construction of interactive dashboards using Power BI, integrating electronic health record and administrative data; (3) Internal validation with the multidisciplinary team and adjustments based on usability and performance; (4) Training of clinical, administrative, and management teams for interpretation and use of BI tool data.

EXPECTED RESULTS:

The BI tool implementation is expected to enable: Transparency and agility in outcome analysis; Real-time monitoring of clinical indicators; Early identification of deviations and complications;

Reduced response time to adverse events; Resource optimization (shorter length of hospital stay); Automated report generation for care, regulatory, and scientific purposes; and Support for clinical research through curated structured data.

KEYWORDS:

Business Intelligence, Performance indicators, Healthcare quality, Information systems, Bone marrow transplantation, Innovation.

IMPLEMENTATION OF HEMATOPOIETIC STEM CELL TRANSPLANT UNIT IN A PEDIATRIC ONCO-HEMATOLOGICAL HOSPITAL: INSTITUTIONAL EXPERIENCE REPORT

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INTRODUCTION

Institutions relying on external centers for HSCT face logistical barriers that cause delays and family strain. The need to restructure an in-house HSCT unit was identified to ensure institutional autonomy, reduce time between indication and procedure, and promote safer, continuous, and more humanized care.

OBJECTIVE

To report the institutional experience in the process of reopening and implementing the new Autogenous Hematopoietic Stem Cell Transplant Unit in a pediatric onco-hematological hospital.

METHODOLOGY

The HSCT unit was restructured with specialized technical support, a transplant physician and consulting nurse. Structural needs were assessed, protocols reviewed, and team size determined. Nurses developed care pathways based on research and visits. The unit underwent physical adjustments, such as structural modifications, HEPA filters, and furniture replacement. Professionals with experience contributed to multidisciplinary meetings, leading to the creation of protocols for autologous HSCT, updates to manuals, and team training.

RESULTS

The unit reopened with three operational beds, positive pressure, HEPA filters, and continuous monitoring. The nursing team consisted of six bedside nurses and six nursing technicians, divided by shifts. The medical team included two specialists and one oncology fellowship student with on-call physicians. The first patient was admitted in September 2024 for cell collection and, in October, for chemotherapy conditioning and the autologous transplant.

DISCUSSION

The experience shows that restructuring an HSCT unit is possible even in complex institutional contexts. This is done by prioritizing technical planning, integrating qualified professionals, and committing to quality care. Specialized external consulting is essential to map the unit's needs and expedite adjustments. Internal professionals should be involved in protocol development. A phased approach allows for a safe process, strengthening institutional leadership and adherence to established practices.

CONCLUSION

The HSCT Unit reopening is a big step forward for pediatric cancer care, increasing independence and expertise. Next steps include standardizing workflows, expanding the team to use all beds, monitoring outcomes, and eventually offering other HSCT types, starting with autologous transplants, with the goal of becoming a leading pediatric transplant center.

IMPLEMENTING PRIMARY NURSE IN A HSCT AND CELL THERAPY UNIT

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INTRODUCTION:

Hematopoietic Stem Cell Transplantation (HSCT) and Cell Therapy involve complex stages requiring a qualified team, with nurses integral to every step. The nursing work process reflects the philosophy guiding patient care. The Primary Nurse model was chosen to direct care, ensuring nurse autonomy and responsibility. This approach values the nurse as central to patient monitoring, ensuring comprehensive, needs-adapted care.

OBJECTIVE:

To describe the implementation of the Primary Nurse model in an HSCT and Cell Therapy unit.

METHODOLOGY:

The model was presented to unit leadership. An internal survey using a structured questionnaire was conducted with staff nurses, assessing autonomy and care perception. This survey provided an initial diagnosis for decision-making and nurse training. Nurses were then categorized as: Primary Nurse (responsible for care planning and interdisciplinary team liaison), associate nurse, specialist nurse, and lead nurse. Comprehensive theoretical and practical training followed, along with project monitoring.

RESULTS:

Implemented in May 2024, the new model led to improved care quality and continuity due to frequent patient-Primary Nurse interaction. Patient satisfaction increased, fostering trust, improving communication, and strengthening the bond with the Primary Nurse. Professionals gained recognition within the multidisciplinary team, acquired new skills, knowledge, and decision-making autonomy. Quality indicators improved, with reduced adverse events and increased patient satisfaction scores (reaching an "excellence zone").

CONCLUSIONS:

Implementing the Primary Nurse model in the HSCT and Cell Therapy unit positively impacted care quality and patient/professional satisfaction. By restructuring the nursing work process, promoting greater autonomy, responsibility, and a holistic patient view, this approach optimizes care. Preliminary results, including enhanced quality and satisfaction indicators, reinforce the model's effectiveness as a promising path to qualify nursing in high-complexity environments and reaffirm nursing as a fundamental pillar in patient-centered care

MANAGEMENT AND IMPLEMENTATION OF HEMATOPOIETIC STEM CELL COLLECTION BY APHERESIS IN A 100% SUS TRANSPLANT CENTER: AN EXPERIENCE REPORT

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a treatment indicated for various diseases, significantly impacting patient prognosis[1]. Hematopoietic progenitor cells (HPCs) can be obtained from bone marrow aspiration, peripheral blood (apheresis), or umbilical cord blood, with transplantation classified as autologous, allogeneic, or syngeneic. In Minas Gerais, HPC collection by apheresis is often performed by outsourced blood centers, limiting access and prolonging waiting times for the procedure. Implementing this collection within the SUS Transplant Centers themselves arises as a strategic alternative to optimize the process, reduce hospitalizations, and minimize risks related to waiting[2]. Nursing roles are pivotal in this context, highlighting the leadership and autonomy of transplant nurses in managing HPC collection and transplantation processes[3].

OBJECTIVE:

To report the professional experience of transplant nurses in leading and implementing the HPC collection process via apheresis at a 100% SUS-funded Transplant Center in Minas Gerais.

METHOD:

This is an experience report by transplant nurses on the implementation of HPC apheresis collection in a 100% SUS-funded Transplant Center located in Minas Gerais.

RESULTS:

The process began in June 2023, with nurses overseeing the contractual logistics with suppliers of materials and laboratory services, coordinating between departments (Transfusion Agency, Antineoplastic Pharmacotechnics, Laboratory, and Biological Materials Transport), selecting staff for the fresh collection team, and managing financial planning. After completing theoretical and practical training provided by the company responsible for the apheresis machine, the nurses developed a Standard Operating Procedure (SOP) for the multidisciplinary team and conducted training sessions for the nursing team. Additional SOPs were created, such as for the infusion of fresh HPCs in autologous HSCT and the use of Vinorelbine for chemomobilization. In October 2024, the first hospitalization involving both HPC collection and infusion in a single admission was carried out, reducing hospital stay duration and promoting financial sustainability. By May 2025, seven collections had been completed.

CONCLUSION:

The implementation of the HPC apheresis collection process highlighted the leading role and decision-making autonomy of the transplant nurse in the planning, execution, and management of care. This role contributed to enhanced care quality, humanization of the process, risk reduction, and institutional sustainability.

KEYWORDS: Hematopoietic Stem Cell Transplantation; Bone Marrow Transplantation Nursing; Health Management.

NURSES PERCEPTION OF CLINICAL SEVERITY AND COMPLICATIONS IN PATIENTS UNDERGOING BONE MARROW TRANSPLANTATION

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INTRODUCTION:

Patients undergoing bone marrow transplantation (BMT) are at high risk for clinical complications, with febrile neutropenia being one of the most frequent and potentially severe events. Mortality can be high if prompt intervention is not provided. During BMT, patients undergo high-intensity chemotherapy and/or radiotherapy regimens, resulting in profound immunosuppression and myelosuppression, increasing the risk of bacterial, fungal, and viral infections. Nursing plays a key role in the early recognition of clinical signs and timely decision-making, especially in high-risk scenarios for sepsis. Understanding how nurses perceive patients' clinical severity and the complications experienced during hospitalization is essential to strengthen safety strategies, interprofessional communication, and clinical surveillance.

OBJECTIVE:

To analyze nurses' perception of the clinical severity of patients hospitalized in the BMT unit and to describe the main care-related complications, with a focus on febrile neutropenia.

METHODOLOGY:

Descriptive, qualitative study conducted with nurses working in the BMT unit of a large hospital in southern Brazil. Data collection was carried out through semi-structured interviews addressing topics such as perception of clinical severity, challenges in recognizing complications, and strategies used to manage febrile neutropenia. The data were analyzed using Bardin's Content Analysis technique.

RESULTS:

Participants reported that the perception of clinical severity is strongly influenced by subtle signs such as changes in hemodynamic patterns, decline in general condition, and progressive prostration, particularly in neutropenic patients. Isolated fever was recognized as a critical marker, triggering immediate actions. Proactive behavior, clinical experience, and familiarity with patient profiles were identified as key factors for the early recognition of complications. Effective communication with the medical team and the presence of care protocols were also highlighted as facilitators of safe care.

CONCLUSION:

Nurses clinical perception is a key element in managing complications in patients undergoing BMT, especially in the context of febrile neutropenia. Investment in continuous training, well-established clinical protocols, and strategies that promote active listening and recognition of nursing experience can strengthen clinical surveillance and contribute to safer patient outcomes. Prevention, early detection, and proper management of febrile neutropenia are fundamental to the success of bone marrow transplantation, requiring a well-trained multidisciplinary team, with a central role played by the nurse.

KEYWORDS:

Transplant nursing; Febrile neutropenia; Clinical perception; Patient safety; Complications in BMT.

NURSING CARE FOR JEHOVAH'S WITNESS PATIENTS: CARE PLANNING BASED ON RESPECT FOR RELIGIOUS CHOICES

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INTRODUCTION:

Humanized care requires the healthcare team to respect patients' beliefs and values, especially when these directly influence clinical decisions. This case report describes the nursing care experience of a pediatric Jehovah's Witness patient with a severe hematological disease who had a clinical indication for blood transfusion, which was refused for religious reasons. In onco-hematological contexts, where invasive interventions and blood product support are common, respecting patient autonomy while ensuring clinical safety are fundamental pillars of care practice.

OBJECTIVE:

To share the experience of nurses in developing and implementing an individualized care plan for Jehovah's Witness patients admitted to a specialized unit for hematological diseases and bone marrow transplantation (BMT).

METHOD:

This is a qualitative clinical case report based on the nursing team's care experience while treating a pediatric patient identified as a Jehovah's Witness undergoing a chemotherapy cycle. The patient was hospitalized in a high-complexity private hospital in the city of Niterói, state of Rio de Janeiro, a national reference center for BMT in Brazil.

RESULTS:

Continuous monitoring of vital signs and peripheral perfusion; prescription of iron, erythropoietin, supplementation with vitamin C, folic acid, and vitamin B12. The use of Romiplostim was indicated as a support strategy if platelet counts approached 50,000/mm^3; conservative practices for laboratory blood collection with reduced volume; family-centered care, teaching the family to safely handle the PICC catheter to ensure treatment efficacy at home; use of low-flow oxygen therapy and strict bleeding control; psychological and spiritual support, with the presence of a representative from the religious congregation; play therapy; meticulous documentation of all nursing actions and clinical progress.

DISCUSSION:

The individualized care plan included measures to minimize blood loss and prevent anemia, such as strict control of laboratory tests, nutritional support, and continuous clinical monitoring. Care was based on ethical principles, active listening, and individualized planning.

CONCLUSION:

Nursing care planning for Jehovah's Witness patients should consider not only clinical needs but also spiritual and ethical aspects. Respect for autonomy, combined with knowledge of therapeutic alternatives, strengthens the humanized and ethical practice of nursing. The importance of patient autonomy, the use of safe therapeutic alternatives, and multiprofessional dialogue is emphasized. This report reinforces the relevance of nursing training for culturally specialized care.

KEYWORDS:

Nursing; Jehovah's Witnesses; Bioethics.

NURSING CARE FOR NECROTIZING FASCIITIS AFTER HEMATOPOIETIC STEM CELL TRANSPLANTATION: A CASE REPORT

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INTRODUCTION:

Necrotizing fasciitis (NF) is a rare, aggressive infection that affects subcutaneous tissue and superficial fascia, causing destruction and necrosis of the affected tissue with high morbidity and mortality rates.

OBJECTIVE:

To describe the nursing care provided to a patient undergoing allogeneic hematopoietic stem cell transplantation (HSCT) with FN. Method: A case report with data collected through the electronic medical record review. The study was submitted to the Research Ethics Committee, and the patient signed the Free and Informed Consent Form.

RESULTS:

A 17-year-old female patient diagnosed with myelodysplastic syndrome underwent haploidentical HSCT, a sex-mismatched, ABO-compatible. The conditioning regimen was the FUCHS protocol. GVHD prophylaxis consisted of low-dose post-transplant cyclophosphamide with mycophenolate mofetil and tacrolimus. It was infused with 2.2x106 CD34+ from a bone marrow source. On day +9, she began to experience pain and edema in the left lower limb, with rapid progression. Computed tomography identified increased volume and fluid sheets in the soft tissues and gas bubbles in the muscle belly and sheath of the left rectus femoris muscle. She developed severe septic shock requiring vasopressors, invasive mechanical ventilation, and hemodialysis. In the serous collection drained during debridement, gram-negative and gram-positive bacilli were identified. Broad-spectrum antibiotics were instituted. Four surgical interventions were necessary, and a vacuum dressing was used for four weeks. The lesion was measuring approximately 15 x 15 cm, with exposed muscle fascia, subcutaneous tissue, and drainage of odorless sero-purulent secretion. Care included: clean the lesion with 0.9% saline solution; application of 0.1% Polyhexanide aqueous solution (PHMB) for 15 minutes; application of low-power laser with RED/InfraRED wavelength and energy of one Joule; application of skin protective spray; covering with acetate fabric impregnated with dialkyl carbamoyl chloride (DACC) and non-adherent cellulose dressing impregnated with fatty acids; covering impregnated with silver alginate in the cavity area and secondary covering with gauze, dressing and bandage. Secondary dressing was changed once a day or more, if necessary, while the primary dressing was changed every two days. The lesion improved, with healing by secondary intention. Discharged from the hospital on Day+79, maintaining outpatient dressing changes, three times a week. The lesion completely healed after five months of treatment.

CONCLUSION:

The correct indication of special dressings, the application of low-frequency laser, continuous monitoring, and the provision of excellent nursing care allowed the associated damages to be minimized. This case report highlights the importance of careful clinical surveillance performed by nurses in HSCT, identifying and intervening early, and in the continuity of care.

KEYWORDS: Necrotizing fasciitis, Hematopoietic Stem Cell Transplantation, Nursing Care.

NURSING CARE FOR PATIENTS UNDERGOING HAPLOIDENTICAL HEMATOPOIETIC STEM CELL TRANSPLANTATION WITH SINUSOIDAL OBSTRUCTION SYNDROME - CASE REPORT

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INTRODUCTION:

Sinusoidal obstruction syndrome (SOS), also known as hepatic veno-occlusive disease (VOD), is a potentially fatal complication of hematopoietic stem cell transplantation (HSCT). It is associated with the conditioning regimen, primarily myeloablative, which causes damage to the endothelium of hepatic sinusoidal capillaries. This syndrome manifests itself clinically with fluid retention, weight gain, painful hepatomegaly, and jaundice. Patients may present cases that resolve quickly, or severe cases that can lead to multiple organ failure. It is essential for the nursing team to be trained in how to identify the initial manifestations to mitigate severe forms, and for this, they need to be aware of the clinical manifestations and the main risk factors.

OBJECTIVE: To describe the case of a patient with SOS/VOD treated in a public HSCT service.

METHOD: It is a case report.

RESULTS:

A 42-year-old man was diagnosed in December 2024 with Ph+ acute lymphoblastic leukemia and underwent the BFM NCRI protocol associated with Imatinib. After achieving complete response, the patient was referred for HSCT and admitted at the inpatient unit in April 2025 with a haploidentical donor. At that time, he had HCT-CI: 3 (hepatic steatosis). The patient was conditioned with the reduced-intensity protocol FLUMEL+TBI200 and received immunosuppressants including Cyclophosphamide post-HSCT, Cyclosporine, and Mycophenolate mofetil. For SOS/VOD prophylaxis, the patient received ursodeoxycholic acid. On day +13 post-HSCT, the patient began to experience unresponsive weight gain to diuretics, worsened renal function, and an increase in total serum bilirubin levels due to direct bilirubin. Supportive measures of fluid and sodium restriction, more frequent monitoring of abdominal circumference and weight, and daily laboratory assessment of liver function, in addition to routine exams, were adopted. On day +15, associated with neutrophil engraftment, the patient experienced clinical deterioration characterized by worsened respiratory patterns interpreted as pulmonary congestion and acute renal failure, requiring renal replacement therapy and ventilatory support in the ICU. The patient did not receive Defibrotide due to unavailability in the service. On day +22, following clinical and laboratory improvement, the SOS/VOD was considered resolved. On day +25, the patient was discharged and referred to the Day Hospital.

CONCLUSION:

The nursing team's role is fundamental in a case of SOS/VOD to identify the first signs. Therefore, the team's training must be meticulous and ongoing to achieve the level of excellence required in HSCT services.

KEYWORDS: Hepatic veno-occlusive disease; nursing care; bone marrow transplantation

NURSING CARE FOR PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a treatment option for oncological and hematological diseases. It is an intravenous infusion of hematopoietic progenitor cells to restore a patient's bone marrow and immune system. The aggressiveness of this treatment and the patient's physical and emotional vulnerabilities make specialized nursing care necessary to promote post-transplant recovery.

OBJECTIVE:

To describe the main points of concern regarding nursing care provided to patients undergoing HSCT.

METHOD:

This is a literature review with a narrative approach.

RESULTS:

HSCT nursing care encompasses different areas, from infection prevention to monitoring early and late complications, with primary focus on patient's security and well-being, crucial to procedure's success. Nursing practice in infection prevention involves strict measures, highlighting hands sanitizer education (Figure 1) directed to multidisciplinary teams, patients, and families. Nurses are also responsible for central venous catheters care, which includes insertion site inspection, catheter dressings change, medication administration, and training and continuing education of the multidisciplinary team, aiming to reduce infections and devices loss due to occlusion or traction. Environmental care, including daily and terminal cleaning, must be planned with the environmental services team, ensuring access restriction to the Inpatient Unit as a measure of biosecurity. Personal hygiene is fundamental in patient's educational process conducted by the nursing team, being indispensable the caregiver engagement and support during more vulnerable moments. Basic nursing cares, as vital signs monitoring, are important to early and timely detection of complications. Daily skin and mucous membranes inspection is necessary due to chemo/radiotherapy side effects and treatment-related immunosuppression, making patients more susceptible to serious injuries. The pain evaluation is a critical care once can indicate mucositis, colitis, and other complications. The use of scales, as the numerical one (Figure 2), assists the nursing team in assessment and helps in early necessary measures implementation. Research and clinical practice emphasize the importance of monitoring hydration and nutrition intake to prevent dehydration or excessive weight gain. Cares with drug therapy and monitoring laboratory tests are essential to prevent and control Sinusoidal Obstruction Syndrome.

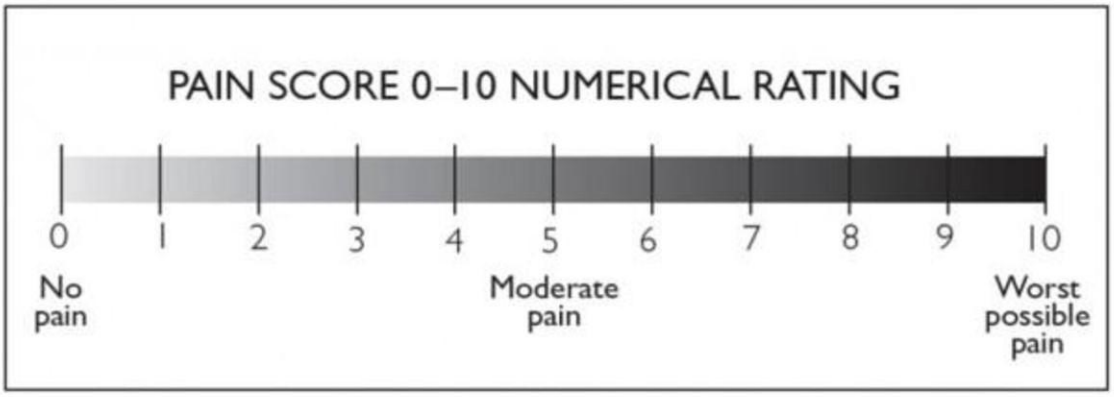
CONCLUSION:

HSCT patients' assistance quality depends on the capacity of the nursing team to provide individualized patient care. For this purpose, the professional must possess technical and scientific knowledge that enables effective nursing care planning, contributing to the patient's quality of life throughout the treatment.

KEYWORDS: Nursing Care; Hematopoietic Stem Cell Transplant; Professional Practice.

Figure 1 – Hands sanitizer education

Figure 2 – Numeric Pain Rating Scale



NURSING CARE IN CAR-T CELL THERAPY: EXPERIENCE REPORT FROM A PRIVATE CELLULAR THERAPY CENTER IN SÃO PAULO

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INTRODUCTION:

CAR-T cell therapy has emerged as a promising alternative for patients with refractory hematologic malignancies. However, its clinical application involves significant care complexity, requiring intensive monitoring and the management of severe adverse events such as cytokine release syndrome (CRS), neurotoxicity, and neutropenia. In this context, nursing care is essential to ensure both the safety and effectiveness of the treatment.

OBJECTIVE:

To report the experience of the nursing team at a cellular therapy center in a private hospital in São Paulo, Brazil, in providing care to patients undergoing CAR-T cell infusion.

METHODS:

This is an experience report based on the clinical practice of the nursing team, including a retrospective analysis of clinical data collected between 2020 and 2025.

RESULTS:

A total of 31 CAR-T cell infusions were performed during the study period: 71% (22) as part of clinical trial protocols and 29% (9) for commercial use. Among clinical trial patients, the most prevalent diagnosis was multiple myeloma (41%; n=9), followed by non-Hodgkin lymphoma (32%; n=7), acute lymphoblastic leukemia (18%; n=4), and chronic lymphocytic leukemia (9%; n=2). In the commercial group, 88.9% (n=8) had non-Hodgkin lymphoma and 11.1% (n=1) had multiple myeloma. A progressive increase in the number of infusions was observed over the years, with a significant rise from 2023 onward. The most common adverse events were: Cytokine release syndrome (CRS): 77% (n=24); Neurotoxicity: 26% (n=8); Neutropenia: 42% (n=13). The growing number of procedures required the enhancement of nursing protocols, particularly in the early identification of CRS, neurological monitoring, and administration of supportive therapies such as tocilizumab. The nursing team played a central role in continuous clinical surveillance, patient and family education, and the training of support staff, including intensive care and outpatient nurses, ensuring continuity of care and patient safety throughout the therapeutic process. Conclusion: The increasing adoption of CAR-T cell therapy has required ongoing training of the nursing team, with a focus on the recognition and management of major adverse events. The center's experience highlights the importance of coordinated nursing care, well-established protocols, and multidisciplinary training to ensure the safe and effective implementation of this innovative therapeutic modality.

KERYWORD: CAR-T cell therapy, Oncology nursing, Adverse events

NURSING INTERVENTIONS FOR VOLUME PRESERVATION AND BLOOD LOSS REDUCTION IN JEHOVAH'S WITNESS PATIENTS UNDERGOING BONE MARROW TRANSPLANTATION: A CASE REPORT

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INTRODUCTION:

Refusing blood transfusion for religious reasons represents an ethical and clinical challenge, especially in the context of Bone Marrow Transplantation (BMT), where cytopenia is expected. The practice of bloodless medicine proposes safe and humane strategies to preserve blood volume and prevent complications. In this scenario, individualized, evidence-based care is essential to guarantee patient autonomy and safety.

OBJECTIVE:

To describe the nursing interventions used to prevent blood volume and reduce blood loss in a patient undergoing allogeneic bone marrow transplantation who refused blood transfusion for religious reasons.

CASUISTRY:

Patient J.S.N, male, 71 years old, Witness of Jehovah's, diagnosed with chronic myeloid leukemia, in myeloid blastic phase at diagnosis, undergoing haploidentical allogeneic BMT with reduced intensity conditioning. He signed a formal refusal of blood transfusions and consented to the use of alternative strategies and volume preservation techniques.

METHODS:

This is a descriptive case report study in which the resources used for data collection were anamnesis, physical examination and analysis of medical records at a reference center for BMT. The interventions were categorized as: laboratory collection techniques, bleeding control, hemodynamic monitoring and pharmacological support. The study complied with Resolution 466/12 of the National Health Council.

RESULTS:

During the 44-day hospitalization, the nursing team took actions such as: laboratory collections in pediatric tubes and periodically every 48/72 hours; monitoring vital signs every 2 hours, strict water balance and abdominal circumference measurement; administration of romiplostim; erythropoetin, ferinject, folic acid and tranexamic, as prescribed by the doctor. He had a progressive drop in hemoglobin (minimum value: 4.1g/dL) and platelets (minimum value: 1,000/mm^3), with active bleeding and signs of hemodynamic instability. However, the actions taken and intensive monitoring led to significant improvements in the tests, and he was discharged from hospital with a good clinical evolution. The neutrophil graft took place on D+22 and the platelet graft on D+25 of the BMT.

CONCLUSION:

Nursing action centered on volume preservation strategies and the prevention of blood loss proved to be effective in the clinical stability and recovery of BMT patients without transfusion. This reinforces the importance of well-structured protocols and team training to ensure safe, ethical care that respects patient autonomy.

KEYWORDS:

Bloodless Medical and Surgical Procedures, Bone Marrow Transplantation, Religion.

NURSING MANAGEMENT IN BONE MARROW TRANSPLANT UNITS: STRATEGIES AND CHALLENGES IN MAINTAINING QUALIFIED TEAMS IN HIGH-COMPLEXITY ENVIRONMENTS

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INTRODUCTION:

Bone marrow transplant (BMT) units are high-complexity environments where patient care demands specialized actions, a multidisciplinary approach, and rapid and accurate decision-making. In this context, nursing management plays a strategic role, being responsible for organizing care, promoting continuous education, and ensuring the quality and safety of care. However, challenges such as a shortage of qualified professionals, high staff turnover, and the ongoing need to update clinical protocols hinder this mission.

OBJECTIVE:

To present the main challenges faced by nursing management in maintaining qualified teams in a BMT unit and to describe the strategies used to overcome them, with a focus on achieving excellence in care.

METHODOLOGY:

This is an experience report conducted in the BMT unit of a large hospital, based on data collected between 2022 and 2024. Care indicators and management practices related to the training and retention of professionals were considered. The variables analyzed included human resources, adherence to clinical protocols, patient safety indicators, and support strategies for the nursing team. Data were collected through institutional documents, such as quality reports, work schedules, and training records.

RESULTS:

The main challenges identified were related to human resources management: a shortage of specialized professionals, high turnover, and difficulty in retaining experienced nurses. The absence of specialized training programs in the region exacerbates this shortage, making continuous internal training essential. The complexity of care, particularly for immunosuppressed patients, requires both technical and behavioral competencies, as well as effective communication between shifts—critical to ensuring continuity and safety in care. Among the strategies adopted were: The development of a structured internal continuing education program with support from the infection control service, the patient safety center, and the medical team; Integration schedules for new staff members, including mentorship from experienced professionals; A gradual and safe process for assuming patient care responsibilities; Encouragement of specialized training;Initiatives for valuing the staff and promoting active listening.

CONCLUSION:

Nursing management in BMT units requires leadership, technical knowledge, and strategic vision to maintain well-prepared and motivated teams. Strategies such as continuous education, effective communication, and professional appreciation are fundamental to ensuring safe, high-quality care for transplant patients. Facing these challenges through planning and innovation is essential to strengthening care delivery and achieving excellence in the services provided.

KEYWORDS: Nursing management, bone marrow transplant, multidisciplinary team.

PARTICIPATORY DEVELOPMENT OF A NURSING PROTOCOL FOR ADMINISTRATION AND MANAGEMENT OF COMPLICATIONS IN PATIENTS USING BISPECIFIC ANTIBODIES IN HEMATOLOGY

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Bispecific monoclonal antibodies (BsAbs) represent a therapeutic innovation in the treatment of hematologic malignancies, particularly multiple myeloma and certain types of lymphoma. Their mechanism of action enables the simultaneous engagement of two cellular targets, promoting greater clinical efficacy. However, the occurrence of serious adverse events—such as cytokine release syndrome and neurotoxicities—requires specialized nursing team preparation to ensure safe care delivery. Continuing education and professional training are key strategies to maintain care quality. This study aimed to develop an institutional nursing protocol for the administration and management of complications associated with the use of BsAbs in hematologic patients. It is a qualitative, participatory study based on Convergent Care Research (CCR). The study included eight nurses working in the bone marrow transplant unit of a large private hospital in southern Brazil. The methodology involved three rounds of structured discussions with participants to gather clinical experiences, identify care gaps, and propose care strategies. Thematic analysis allowed for the systematization of information and collaborative validation of the protocol. Key outcomes included the definition of eligibility criteria for BsAb administration, standardization of preparation and infusion procedures, monitoring of critical clinical signs, interventions for adverse events, and multiprofessional response flows. The participatory construction process empowered nursing leadership and consolidated evidence-based practices. It is concluded that the protocol contributes to the improvement of care quality, enhances patient safety, and can be replicated in other hematology settings. Future studies should assess the impact of its implementation on clinical and operational outcomes. Thus, the safe use of BsAbs depends on well-trained healthcare teams—especially nurses, who are responsible for administration, monitoring, and intervention in response to complications. The development of evidence-based care protocols is essential to ensure treatment effectiveness and patient safety.

KEYWORDS: Bispecific antibodies; Multiple myeloma; Oncology nursing

PERIPHERALLY INSERTED CENTRAL CATHETER EXPERIENCE IN HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is an important treatment of several hematological and oncological diseases. During this process, the use of a central venous catheter becomes essential. The peripherally inserted central catheter (PICC) is an excellent option. It is a non-invasive device implanted patient's bedside without anesthesia and therefore outside surgical center. It ensures safe administration of medications and blood products, is positioned in a patient-friendly location, and is easy to manage. Because of that, it has a low risk of complications during and after insertion, mainly infections. According to Resolution nº 258/2001 of the Federal Nursing Council, a qualified nurse is responsible for catheter implantation.

OBJECTIVE:

To describe the experience of nurses systematically implanting PICC in patients undergone HSCT at a public health service.

METHOD:

It is a case series of adults' patients undergone allogeneic HSCT from March 2022 to December 2024 that used PICC. The devices were implanted by five nurses who evaluated patients' medical history and performed the procedure using an ultrasound transducer and the modified Seldinger technique, after receiving practical and theoretical training.

RESULTS:

Fifty-seven catheters were implanted in patients. Average age of the patients was 44 years old and there was a small predominance of men (59.7% men and 40.3% women). The main diagnosis was acute myeloid leukemia (44.8%) and the main transplant modality was haploidentical transplant (50%). The right upper limb was the primary insertion site (75.4%). All catheters were implanted using the basilic vein, with the assistance of ultrasound. Fifty (87.7%) PICCs had their positions confirmed and were released by the physician team after patients underwent chest x-ray, while seven (12.3%) were released by the nurses during the procedure using the 3CG navigation system. The average of catheter duration was 140 days, with a variable range of 20 to 500 days, although two patients had catheters during this research. The catheters were used for the infusion of crystalloid, blood components, HSCT cell infusion, and for the collection of blood to laboratory tests. The main reason for removing the catheters was the treatment end (70.2%), followed by suspected catheter-related infection (17.5%), and there was no removal due to occlusion or traction (Tabel 2).

CONCLUSION:

In the context of HSCT, the PICC has been shown to be safe and effective. Planning and implantation can be made at an outpatient facility, rather than at a surgical center. The complication rate is low as long as the PICC is under qualify nurse responsibility in a continuing education program.

KEYWORDS:

Hematopoietic Stem Cell Transplantation; Peripherally Inserted Central Catheter; Nursing.

TABLE 1 – Data relating to PICC (n = 57)

Variable	Category	n	(%)
Time of use (days)	Min-Max Average	20 -500 140,3	- -
Local	Right upper limb Left upper limb	43 14	75,4 24,6
Reasons for removal	Suspect of infection Catheter-related infection confirmed Trombosis End of treatment Catheter in use	10 2 3 40 2	17,5 3,5 5,3 70,2 3,5

RECRUITMENT OF NURSING JUDGES FOR CONTENT VALIDATION IN A METHODOLOGICAL STUDY: EXPERIENCE REPORT

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1 Bone Marrow Transplant Center- National Cancer Institute
2 Program

INTRODUCTION

Hematopoietic Stem Cell Transplantation (HSCT), especially in the allogeneic modality , is a complex procedure that requires specific nursing care and patients often have a compromised venous network due to multiple previous treatments, with an increased risk of infections and bleeding due to aplasia. The use of a central venous catheter is essential for the safe infusion of stem cells, immunosuppressive drugs and collection of blood samples for serum dosage. This dosage is essential for the prevention of Graft-versus-Host Disease, which is one of the main complications in the first 100 days after allogeneic HSCT.

OBJECTIVE:

to report the authors' experience in recruiting expert judges to validate the content of a care protocol aimed at collecting samples for serum dosage of immunosuppressants administered intravenously in patients undergoing allogeneic HSCT. Methodology: This is an experience report with a qualitative approach, classified as exploratory and descriptive. The first stage of recruiting expert judges consisted of actively searching for resumes on the Lattes Platform, considering nursing professionals working in the HSCT area. Thirty-two resumes were analyzed, and those that did not have practical experience in the area or belonged to other professional categories were excluded. Next, invitations were sent through the platform itself, respecting the daily limit for sending messages. As a complementary strategy, the network sampling technique (snowball) was used, with contacts made via the WhatsApp® application. Interested professionals received the link to the Free and Informed Consent Form (FICF) on Google Forms® , followed by the sociodemographic and protocol evaluation questionnaires.

RESULTS:

100 invitations were sent. Of these, 45 professionals agreed to participate, but only 30 signed the FICF and contributed to the protocol evaluation. The limitation of sending messages through the Lattes Platform, outdated resumes, and difficulty in accessing professionals' emails were some of the challenges faced. The snowball technique proved to be more efficient in recruiting professionals, allowing professionals from different regions of the country to be reached.

CONCLUSION:

The experience showed that recruiting expert judges for content validation is a laborious step that requires time, planning and the use of different strategies. The combined use of the Lattes Platform and the snowball technique expanded the reach and effectiveness of the recruitment, favoring adherence to the study. The importance of the availability, interest and engagement of the invited professionals is highlighted, which were fundamental to the success of the validation process of the care protocol aimed at the care of patients undergoing allogeneic HSCT.

KEYWORDS: Catheters; Hematopoietic Stem Cell Transplantation; immunosuppressants.

RELEVANT VARIABLES FOR DECIDING ON THE REMOVAL OF MEDIUM- OR LONG-TERM CENTRAL VENOUS CATHETERS IN POST-ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANT PATIENTS: EXPERT OPINION

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1. Federal University of Rio de Janeiro

INTRODUCTION:

Patients undergoing allogeneic hematopoietic stem cell transplantation (allo-HSCT) require a central venous catheter (CVC) for therapeutic support. After hospital discharge, the CVC remains essential due to the high risk of acute and chronic complications, particularly during the first three months. Therefore, maintaining the CVC during outpatient follow-up is justified. It is necessary, however, to establish clinical criteria to guide the optimal timing for CVC removal, balancing the risks and benefits of its continued use.

OBJECTIVE:

To identify the variables considered relevant by experts for deciding on the removal of medium- or long-term CVCs in post-allo-HSCT patients.

METHOD:

An integrative literature review and expert validation of the identified variables. The literature review was conducted across three databases, including full-text articles published up to 2022, related to post-allo-HSCT complications. Articles outside the scope, in non-Latin languages, or classified as grey literature were excluded. The search, performed in March 2023, followed the PICo acronym. A total of 315 articles were retrieved, with 267 included. Evidence on complications was grouped by similarity. Subsequently, a panel of 20 experts (physicians and nurses experienced in HSCT) validated the variables through a dichotomous scale questionnaire. An open-ended question was also included to gather clinical insights. Variables were assessed using the Content Validity Ratio (CVR). Variables considered essential were those with a CVR ≥ the critical CVR for 20 participants, which is 0.500. The study was approved by a Research Ethics Committee.

RESULTS:

The review yielded 60 grouped complication categories, from which 123 dichotomous questions emerged. The questionnaire was completed in October 2024. Most participants were female nurses working in public institutions. The average age was 47 years, with an average of 15 years of experience in allo-HSCT. The open-ended question yielded 24 evidence groups, all of which were already included in the closed questions, except for signs of inflammation and catheter-related infection. The analysis of the closed questions identified 47 variables with a CVR ≥ 0.500. Among them: clinical frailty, severe or refractory GVHD, cytopenias, organ dysfunctions, and poor treatment adherence.

CONCLUSIONS:

The study identified a set of factors that, according to specialists, influence the decision to maintain or remove the CVC in transplant patients. Time since allo-HSCT was not a determining factor, as commonly believed. Experts emphasized the patient's current clinical condition, catheter-related factors, and therapeutic forecasts as key decision-making elements. Careful evaluation of these elements is essential to avoid both premature removal and unnecessary maintenance of the device.

KEYWORDS: Hematopoietic Stem Cell Transplantation; Allografts; Central Venous Catheters

SUICIDAL IDEATION AMONG PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION: A CROSS-SECTIONAL STUDY

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INTRODUCTION:

Suicidal ideation refers to the thought of an intense desire to die and the making of certain decisions, such as the formulation of a detailed plan of actions and methods that lead to suicide. People with oncological diseases are twice as likely to commit this act when compared to the general population, due to the stigma that the diagnosis of neoplastic disease carries. Among neoplasms, hematological diseases also stand out, since during Hematopoietic Stem Cell Transplantation (HSCT) a reduction in quality of life is observed, related to chemotherapy treatment, protective isolation and the risks that such therapy poses. As a result, many patients develop mental disorders with a risk of suicidal ideation, which can occur at any stage of the transplant.

OBJECTIVE:

To characterize the sociodemographic and clinical profile of patients undergoing hematopoietic stem cell transplantation who presented suicidal ideation.

METHODS:

This is a quantitative study with a cross-sectional design. Data collection was performed through two questionnaires, one that addressed the epidemiological characteristics (sociodemographic and clinical) related to HSCT and another questionnaire called Self-Reporting Questionnaire (SRQ-20), which investigated Common Mental Disorder and risk for suicidal ideation. The Research Protocol of this study was approved by the Ethics and Research Committee of the Federal University of Rio Grande do Norte under CAAE: 35199320.5.0000.5537.

RESULTS:

Among the 91 study participants, 11 presented Suicidal Ideation (12.0%). Of these, 72.6% were between 20 and 59 years of age, 72.6% were male and 81.8% declared themselves to be of mixed race. Multiple Myeloma stood out as the underlying disease (54.5%) and they underwent autologous transplant. Among the toxicities presented by the patients, nausea (100%), vomiting (91.0%) and changes in taste (91.0%) were the most cited. Regarding the SRQ-20 variables, 72.7% had a lack of appetite, 91.0% felt nervous, tense or worried and 54.5% had more frequent crying episodes.

CONCLUSION:

This study highlights a current public health problem in a specific population and draws the attention of the multidisciplinary team of Hematopoietic Stem Cell Transplant services to the early identification of signs and symptoms of Common Mental Disorder and Suicidal Ideation. Furthermore, it points to the need for Public Policies that support quality in care for patients with Suicidal Ideation and the conduct of new research.

KEYWORDS: Suicidal Ideation, Pacients; Hematopoietic Stem Cell Transplantation.

THE IMPORTANCE OF THE NAVIGATOR NURSE IN PEDIATRIC BONE MARROW TRANSPLANTATION WITHIN THE CONTEXT OF THE INSTITUTIONAL DEVELOPMENT SUPPORT PROGRAM OF THE UNIFIED HEALTH SYSTEM (PROADI-SUS)

Juliana Araujo De Souza[1]; Natalia Moreno Lamonato Dos Reis[1]; José Ulysses Amigo Filho[1]; Augusto Magno Tranquezi Cordeiro[1]; Priscila Tavares Musqueira[1]; Rafaella Louize Francisco Gomes Moia[1]; Caroline Souza Gomes Bernardo[1]; Lais da Silva Crochik[1]

1. BP – A Beneficência Portuguesa de São Paulo

INTRODUCTION:

Pediatric bone marrow transplantation (BMT) is a complex procedure that requires specialized care from a multidisciplinary team. In the contex of the Unified Health System (SUS), especially through the Institutional Development Support Program of SUS (PROADI-SUS), aims to ensure humanized, efficient, and safe care for these patients. In this scenario, the role of the navigator nurse has become essential to optimize care, promote effective communication among all team members, with the patient/family, and ensure continuity of treatment.

OBJECTIVE:

This work aims to highlight the importance and role of the navigator nurse in pediatric BMT and within the PROADI-SUS context, emphasizing their role in care, guidance to families, and promoting safer and more humanized assistance.

METHOD:

We conducted a literature review and a qualitative analysis using the Net Promoter Score (NPS) with transplanted patients at one of the transplant centers within PROADI-SUS. This allowed us to assess the relevance of the navigator nurse's role in clinical practice, care management, and communication among the multidisciplinary team, patients, and families. The methodology sought to demonstrate how this professional contributes to improving the patient experience in the context of pediatric BMT.

RESULTS:

Studies indicate that the navigator nurse acts as a facilitator in communication between the multidisciplinary team, patient, and family, promoting more integrated and patient-centered care throughout their journey (as exemplified in Figure 1). Recently, COFEN (Federal Nursing Council) standardized the role of the Navigator Nurse through Resolution No. 735 of January 17, 2024, which regulates the activities of the Navigator Nurse and the specialized clinical nurse. Their role contributes to reducing complications, improving treatment adherence, and increasing family satisfaction (Figures 2 and 3 demonstrate satisfaction through NPS). Within PROADI-SUS, this function reinforces the importance of strategies that promote humanization and integrality of care.

CONCLUSION:

The results reinforce the importance of the navigator nurse's role as an essential agent in qualifying care and humanizing the patient journey, being indispensable in caring for pediatric patients undergoing BMT, especially within the PROADI-SUS context. Their actions enhance care coordination, promote humanization, and improve clinical outcomes. Investing in the training and appreciation of this professional is fundamental to improving healthcare assistance and ensuring a safer and more welcoming experience for children and their families.

KEYWORDS: Navigator Nurse; Pediatric BMT; PROADI-SUS

FIGURE 1 - Patient Journey Flowchart

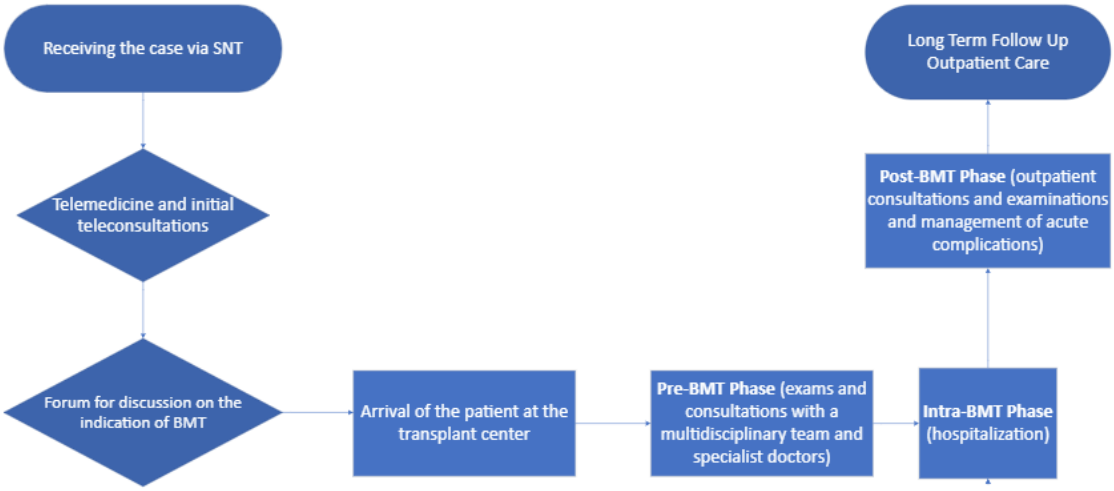
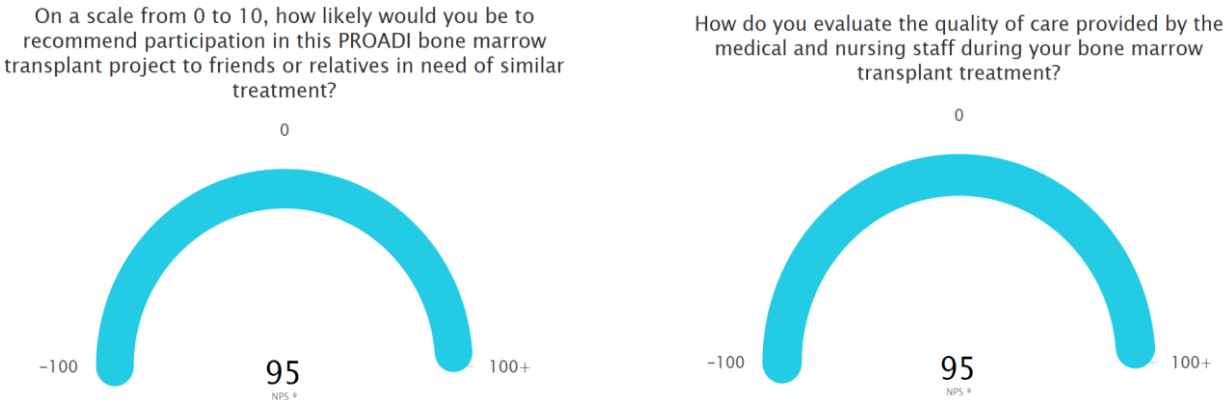
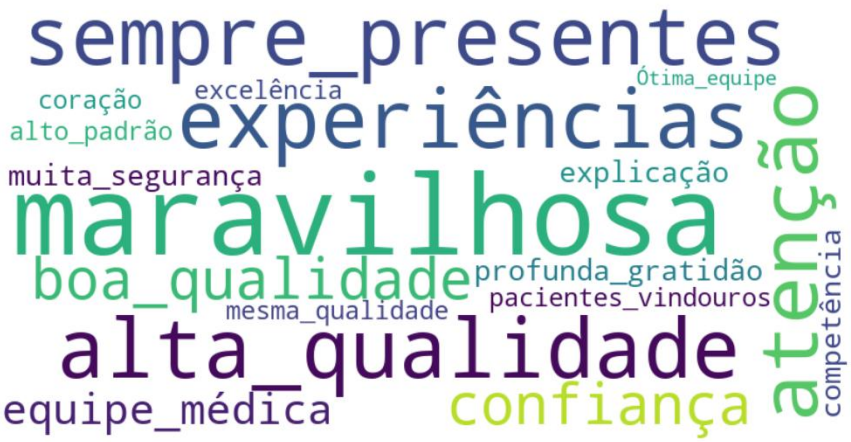


FIGURE 2 - Net promoter score



- Figure 2 - Net Promoter Score

FIGURE 3 - Word cloud generated from the Net Promoter Score



THE ROLE OF NURSES IN THE BONE MARROW TRANSPLANT SECTOR FOR JEHOVAH'S WITNESS PATIENTS: EXPERIENCE REPORT

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INTRODUCTION:

Bone Marrow Transplantation (BMT) is a highly complex procedure which, in many cases, requires blood transfusions to ensure patient survival and therapeutic success. However, Jehovah's Witness patients, for religious reasons, refuse the administration of blood components, which imposes ethical, legal and care challenges on health professionals, especially nurses, who follow the patient continuously and directly. The Nursing Code of Ethics and Brazilian legislation ensure the right to autonomy and therapeutic refusal, as long as the patient is aware and duly informed, and it is the professional's duty to respect these decisions. In this context, it is essential that nurses are trained to deal with situations of conflict between clinical practice and patients' individual values, adopting alternative and safe strategies for care.

OBJECTIVES:

To report on the work of nurses with Jehovah's Witness patients undergoing bone marrow transplants who do not receive blood transfusions, highlighting the challenges faced when refusing blood transfusions for religious reasons.

METHODS:

This is an experience report from the BMT inpatient unit of a private hospital in São Paulo over a six-month period.

RESULTS:

Caring for Jehovah's Witness patients undergoing BMT required an interdisciplinary approach based on respect for the patient's autonomy and rights. The nurse's role stood out in the implementation of alternative strategies to transfusion, such as blood conservation techniques; administration of hemostatic agents, erythropoietin, iron supplements, vitamin B12 and folic acid; strict monitoring of signs of bleeding and hemodynamic conservation. In addition, therapeutic communication was essential to promote person-centered care, fostering a bond of trust between the health team, the patient and their family.

CONCLUSION:

Nursing care for Jehovah's Witness patients requires, in addition to clinical knowledge, ethical sensitivity, respect for autonomy and an understanding of alternative practices to transfusion. It is worth pointing out that, even in the face of the limitations imposed by religious convictions, it is possible to reconcile technical-scientific excellence with the humanization of care, bloodless medical and surgical procedures and bioethical concepts, in order to achieve a favourable outcome with the interdisciplinary team in the bone marrow transplant process.

KEYWORDS: Bloodless Medical and Surgical Procedures, Bone Marrow Transplantation, Nursing.

THE ROLE OF THE NURSE NAVIGATOR IN CARING FOR ADULT PATIENTS IN THE PRE-HEMATOPOIETIC STEM CELL TRANSPLANTATION PHASE: AN EXPERIENCE REPORT

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is the recommended treatment for patients with cancers such as leukemia, lymphoma, multiple myeloma, myelodysplasia syndrome, and myelofibrosis. Its goal is to replace diseased cells with healthy ones. The pre-transplant phase involves multidisciplinary monitoring to assess whether the patient is eligible for this treatment and to prepare them for the upcoming phases, potential side effects, and complications through meetings and the evaluation of test results. The pre-HSCT nurse navigator works in an individualized manner, managing care, providing health education, and assists patients and their families in better understand the hematopoietic stem cell transplant process. Her role in this initial stage is crucial for guiding the patient, monitoring adherence to the multidisciplinary care provided, coordinating hospital admissions, scheduling hematopoietic stem cell mobilization (both autologous and allogeneic), and following up on the results of tests requested for pre-transplant assessment.

OBJECTIVE:

To highlight the importance of pre-HSCT nursing navigation and its impact on the pre-admission process.

METHOD:

Experience report based on activities carried out at the outpatient clinic of the adult Hematopoietic Stem Cell Transplantation Unit of a cancer treatment institution located in southern Brazil.

RESULTS:

The pre-HSCT nurse navigator welcomes the patient following the medical consultation, which continues with guidance on the treatment, hospitalization, instructions for companions and visitors, and information on possible complications and adverse reactions such as graft-versus-host disease (GVHD), mucositis, alopecia, opportunistic infections, etc.is carried out according to the specifics of each treatment, and the patient is referred for multidisciplinary follow-up at the institution with psychologists, nutritionists, dentists, social workers, and infectious disease specialists. Additionally, the navigator reviews the patient's schedule to ensure attendance at all appointments, with absences monitored via telemonitoring. The impact of pre-HSCT navigation also reflects in bed management. Through ongoing monitoring and filling out spreadsheets with patient and treatment information, hospital admissions are scheduled according to bed availability and individual needs.

CONCLUSION:

Pre-HSCT nursing navigation ensures that the patient follows a comprehensive and individualized care pathway, gains a better understanding of the proposed treatment and its adverse effects, and reduces hospitalization time.

KEYWORDS: Nurse Navigator; HSCT; Pre-HSCT Navigation.

THE USE OF NAVIGATION AS A HEALTH LITERACY STRATEGY FOR PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION: A SUCCESSFUL NURSING EXPERIENCE

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INTRODUCTION:

Hematopoietic Stem Cell Transplantation (HSCT) is one of the forms of treatment for numerous malignant diseases. It is a procedure performed in several stages and consists of replacing the recipient's hematopoietic system. In order to provide nursing care in order to identify barriers and act to mitigate them, make referrals, expedite appointments, promote health education, clarify doubts and fears, and prepare the patient in an agile and efficient manner, the nurse navigator is a crucial part of the HSCT service. Following in the footsteps of pioneering countries in patient navigation (NP), Brazil published Law 14,758 in December 2023, the National Policy for Cancer Prevention and Control within the scope of the Unified Health System and the National Program for Navigation of People Diagnosed with Cancer. Health literacy (HL) consists of the ability to receive, assimilate, and understand health information in order to make appropriate health decisions, understand the process in which one is involved, be able to ask questions, trust the processes, and be empowered in the face of facts.

OBJECTIVE:

To report the experience of navigation as a health literacy strategy for patients undergoing HSCT.

METHOD:

PN was instituted in the HSCT service in January 2023 for all patients. After undergoing the medical consultation, the patient goes to the consultation with the nurse navigator, where a specific form is filled out to identify the barriers to be worked on and where all the exams and multidisciplinary consultations necessary to prepare for HSCT are scheduled. At this time, the WhatsApp number that is exclusive to navigation is provided. This channel operates from Monday to Friday, from 8 am to 6 pm. During the pre-transplant period, the navigator maintains frequent contact with patients, monitoring attendance at scheduled appointments and exams, answering questions, rescheduling appointments, sending reminders about exam times and preparations, and providing various communications from the service. During hospitalization, the navigator visits patients' beds immediately after admission and advises them that the communication channel will be open for communication. After HSCT, the navigator remains available to patients, but waits to be contacted by them to act, if necessary.

RESULTS:

Contact via WhatsApp allowed communication through audio messages, which brought illiterate patients closer together and enabled communication with them. The quick response time, around 30 minutes, ensured agility in resolving demands. The bond created between the navigator and the patient was strengthened and promoted greater adherence and confidence in the treatment.

CONCLUSION:

The navigation of patients undergoing HSCT contributed to greater LS, adherence to treatment, as well as to the creation of a link between the patient and the health team.

KEYWORDS:

Patient navigation, Health literacy, Hematopoietic stem cell transplantation.

USE OF LOW-LEVEL LASER THERAPY IN THE PREVENTION AND TREATMENT OF SKIN LESIONS IN PATIENTS WITH HEMATOLOGICAL DISEASES IN A BONE MARROW TRANSPLANT UNIT

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INTRODUCTION:

Conditioning, one of the stages of hematopoietic stem cell transplantation (HSCT), performed with high-dose chemotherapy, or even graft-versus-host disease (GVHD), which occurs in allogeneic transplantation, can lead to numerous complications with associated skin manifestations. Therefore, pre-transplant evaluation must be rigorous and include a detailed analysis of the patient's health conditions, aiming to optimize treatment and early identify risk factors that could trigger cutaneous complications.

Low-level laser therapy (LLLT) or low-intensity laser therapy brings many benefits, including reducing the acute inflammatory process and facilitating the organization of collagen fibers and fibroblast production, thereby shortening healing time.

OBJECTIVE:

To describe the implementation of low-level laser therapy as a nursing care strategy for the prevention and treatment of skin lesions in hematological patients with chemotherapy-induced diarrhea or skin involvement resulting from antineoplastic treatment.

METHODOLOGY:

Experience report of nursing care practices developed by a nursing team in a bone marrow transplant unit of a large hospital in southern Brazil.

EXPERIENCE REPORT:

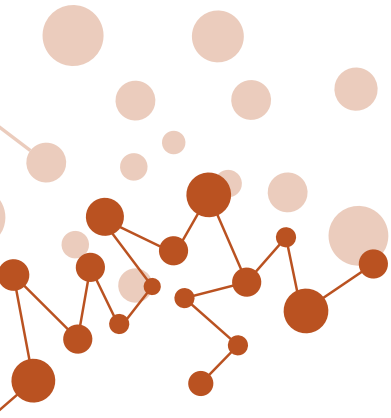
It was observed that during HSCT, patients with skin conditions such as abscesses, onychocryptosis, dermatitis, among others, benefit from the use of LLLT to accelerate the healing process. This enables them to continue hematological treatment and reduces the risk of microbial entry, which can lead to skin infections and even reach the bloodstream, causing systemic and invasive infections.

Based on the nurses' experience, it was found that the application of LLLT led to lesion resolution and restoration of skin integrity in most cases.

CONCLUSION:

The use of low-level laser therapy proved to be an effective and safe strategy in managing skin lesions associated with diarrhea in hematological patients. The adoption of this practice within nursing care broadens care possibilities, promotes patient comfort, and contributes to the prevention of infectious complications. When applied to skin lesions in patients undergoing the HSCT process, LLLT enables better recovery of skin integrity, offering patients comfort, pain reduction, and shorter tissue healing time. The trained nurse acts as a transformative agent of care, adding value to the healthcare provided.

KEYWORDS: Oncohematologic nursing; Low-level laser therapy; Skin lesion; Chemotherapy-induced diarrhea; Bone marrow transplant.



PHARMACY

CLINICAL PHARMACIST INVOLVEMENT IN PRE-HSCT CARE: AN APPROACH TO MEDICATION SAFETY

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INTRODUCTION:

Candidates for Hematopoietic Stem Cell Transplantation (HSCT) face significant risks, including infections, adverse drug reactions, and drug-drug interactions (DDIs). Considering the essential contribution of clinical pharmacists to patient safety, a structured pharmaceutical assessment process was introduced in the pre-transplant phase. This process involves a comprehensive review of home medications, identification of allergies, evaluation of potential DDIs, and continuous medication counseling throughout the HSCT journey.

OBJECTIVES:

To present the findings of pre-HSCT pharmaceutical assessments performed in a specialized care service.

METHODS:

This is a retrospective, observational, and descriptive study conducted from November 2024 to May 2025 with HSCT candidates at a private specialized center in Nova Lima, Minas Gerais, Brazil. Data were obtained through the analysis of standardized electronic pharmaceutical care documentation.. The collected data were quantified and descriptively reported to assess the effectiveness of pharmaceutical evaluation in enhancing medication safety and optimizing patient preparation prior to HSCT.

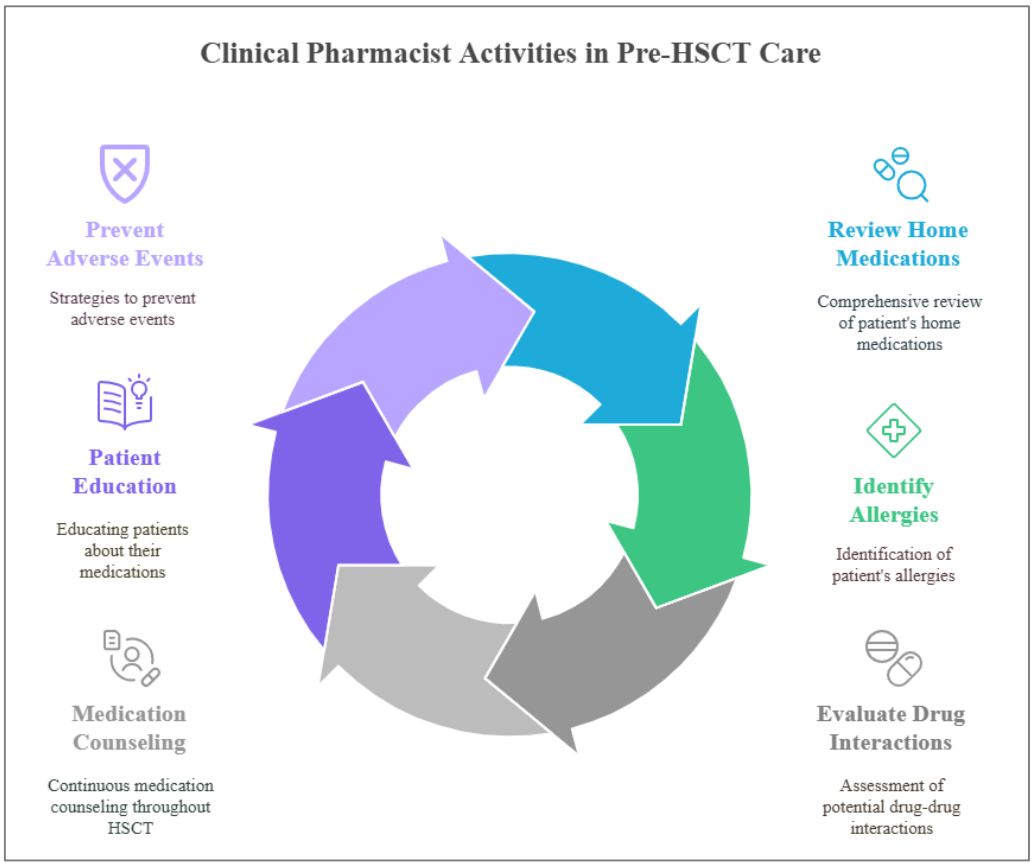
RESULTS:

Data regarding medication reconciliation and analysis of potential DDIs were reviewed, with a focus on drugs used in the conditioning regimen. Among 58 HSCT candidates during the study period, 81% were evaluated by the pharmacist, with 65% indicated for autologous transplant. Medication reconciliation was performed in all assessed patients, and polypharmacy—defined as the regular use of five or more medications—was observed in 43% of patients. Potential DDIs were identified in 46% of patients, with 51% involving interactions between home medications and conditioning chemotherapy. Melphalan was the drug most frequently implicated in these interactions, appearing in 17 cases. The main therapeutic classes involved in DDIs with melphalan were anticoagulants and antihypertensives, potentially increasing the risk of bleeding and hypotension, respectively. Conclusion: Pharmaceutical assessments ensured accurate medication histories and systematic identification of DDIs. In addition to the findings of this study, it is important to highlight that DDIs involving conditioning chemotherapy are well-documented in the literature. For instance: (i) busulfan with aprepitant and azole antifungals; (ii) melphalan with allopurinol, vancomycin, certain antihypertensives, and anticoagulants. Beyond supporting eligibility screening, pharmacists play an active role in patient education, adverse event prevention, and safe preparation for HSCT. The findings highlight the essential role of clinical pharmacists in multidisciplinary teams, supporting patient safety and therapeutic optimization across all phases of the transplant process.

KEYWORDS:

Pharmaceutical Assessment, Medication Safety, Transplantation Condicioning

FIGURE 1


Clinical Pharmacist Activities in Pre-HSCT Care
Prevent Adverse Events
Strategies to prevent adverse events
Patient Education
Educating patients about their medications
Medication Counseling
Continuous medication counseling throughout HSCT
Review Home Medications
Comprehensive review of patient's home medications
Identify Allergies
Identification of patient's allergies
Evaluate Drug Interactions
Assessment of potential drug-drug interactions

CONDITIONING REGIMEN FOR AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION AND THE OCCURRENCE OF ORAL MUCOSITIS

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a potentially curative treatment for a wide variety of diseases. It is a highly complex procedure, the success of which directly involves the work of a multidisciplinary team. Oral mucositis is a common complication during HSCT and the intensity of the conditioning regimen is directly related to the occurrence and severity of mucositis. The pharmacist and the nutritionist have fundamental roles in this process, working to prevent and manage mucositis.

OBJECTIVE:

To describe the profile of patients who have undergone autologous HSCT to date, the occurrence and degree of mucositis, and the protocol used in the conditioning regimen.

CASE SERIES:

Patients undergoing autologous HSCT from December 2021 to April 2025.

METHOD:

Cross-sectional study including patients monitored by a multidisciplinary team upon admission for HSCT. Data were collected from patient assessment and review of electronic medical records. The primary outcome was the prevalence of mucositis, which was classified according to CTCAE v5.0. For descriptive analysis, frequencies, means, medians, and standard deviations were calculated.

RESULTS:

A total of 65 HSCT were performed during the period. The mean age of patients was 58.26 years (31 - 73 years; SD = 9.56), 69.3% were male and their diagnosis were multiple myeloma (95.4%; n = 62) and lymphoma (4.6%; n = 3). The conditioning protocols used were Melphalan 140mg/m2 in 28 patients (43.1%), Melphalan 200mg/m2 in 34 cases (52.3%) and BEAM in 3 patients (4.6%). In total, 53 patients presented mucositis, 73.6% (n=39) of cases were classified as grades 1-2 and 26,4% (n=14) as grades 3-4. When comparing the occurrence of mucositis by type of protocol used, it was observed that only 3 patients who received Melphalan 140mg/m2 presented mucositis grades 3-4 versus 14 patients who received Melphalan 200mg/m2, and none who received the BEAM protocol presented severe mucositis. It was observed that mucositis occurred regardless of the protocol used, but there was a higher prevalence of grade 3-4 mucositis in patients who received Melphalan 200mg/m2 compared to those who received Melphalan 140mg/m2 or BEAM (39% vs. 13% vs. 0%, respectively). Every patient required dietary adjustments and had an increased need for medication, however, only one patient required parenteral nutrition during hospitalization, and this patient received Melphalan 200mg/m2.

CONCLUSIONS:

This data corroborates what is described in the literature, demonstrating that the use of more intense conditioning protocols results in more cases of severe mucositis. This finding reinforces the need to implement mucositis prevention protocols and highlights the importance of multidisciplinary monitoring during all phases of HSCT, especially by the pharmacist and nutritionist, considering the impact of conditioning chemotherapy on the need for dietary adjustments and HSCT complications such as mucositis.

KEYWORDS:

Hematopoietic Stem Cell Transplantation, Bone Marrow Transplantation, Mucositis

DRUG-RELATED PROBLEMS AND PHARMACEUTICAL INTERVENTIONS IN PATIENTS UNDERGOING AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION: THE ROLE OF THE CLINICAL PHARMACIST IN A MULTIDISCIPLINARY TEAM

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a highly complex, potentially curative treatment for a wide variety of hematologic diseases. It is a procedure whose success directly involves the work of a specialized multidisciplinary team, which includes an oncology pharmacist. Pharmaceutical care aims to ensure that drug therapy is properly indicated and that it is more effective, safe, and convenient. The pharmacist in HSCT works to prevent and resolve drug-related problems (DRPs) and provides information to the patient regarding the medications used during HSCT.

OBJECTIVE:

To describe the identified DRPs and pharmaceutical interventions performed, in addition to describing the profile of patients who underwent autologous HSCT at the institution.

CASE SERIES:

patients who underwent autologous HSCT from December 2021 to April 2025.

METHOD:

Descriptive study presenting data from patients undergoing HSCT. Patients were monitored by the clinical pharmacist from pre-HSCT evaluation, case discussion in multidisciplinary rounds, until hospitalization for the procedure and discharge. Data were collected from patient evaluation, follow-up during hospitalization and review of electronic medical records. When problems were detected, the oncology pharmacist intervened with the physician or another health professional responsible for the patient. For descriptive analysis, frequencies, means, medians and standard deviations were calculated.

RESULTS:

Sixty-five autologous HSCT were performed from December 2021 to April 2025. All patients were evaluated and monitored by the pharmacist. The mean age of the patients was 58.26 years (31 - 73 years; SD = 9.56), and 69.3% were male. Patients used a median of 4 medications before transplantation (0 to 15). Most patients were diagnosed with multiple myeloma (95.4%; n = 62) followed by lymphomas (4.6%; n = 3). The conditioning protocol used was Melphalan 140mg/m2 in 28 patients (43.1%), Melphalan 200mg/m2 in 34 patients (52.3%) and Carmustine, Ethoposide, Cytarabine and Melphalan (BEAM) in 3 patients (4.6%). The median hospital stay was 17 days (14 to 36 days), and 1 patient remained hospitalized after April 2025. A total of 299 DRPs were identified during this period. The most common problem was the occurrence of adverse reactions, in 58.8% of cases (n=176), followed by the need to include an additional medication (16.4%;

n=49) and discrepancies in relation to previously used medication (4%; n=12). A total of 82 interventions were performed, and 75.6% (n=62) were accepted after the pharmacist contacted the physician. The most frequent adverse reactions were mucositis (n=46), febrile neutropenia (n=40), nausea and vomiting (n=31), and diarrhea (n=30).

CONCLUSIONS:

The participation of the oncology pharmacist contributed to increase the detection and resolution of drug-related problems and to the safety of the therapy, demonstrating the importance of specialized multidisciplinary monitoring.

KEYWORDS:

Hematopoietic stem cell transplantation, drug-related problems, oncology pharmacist

EVALUATION OF ANTIMICROBIAL CONSUMPTION AND ADHERENCE TO THE FEBRILE NEUTROPENIA PROTOCOL IN A BONE MARROW TRANSPLANT UNIT: A MULTIDISCIPLINARY ANALYSIS

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INTRODUCTION:

Patients undergoing bone marrow transplantation (BMT) are at high risk of developing febrile neutropenia (FN), a severe and potentially life-threatening complication. FN management relies on rapid and appropriate administration of broad-spectrum antimicrobials. Institutional protocols are essential to guide clinical decision-making, standardize care, and improve outcomes. However, their success is strongly dependent on adherence by the multidisciplinary team. Low adherence may delay treatment initiation, contribute to inappropriate antimicrobial use, and increase hospital stays and mortality.

OBJECTIVE:

To evaluate antimicrobial consumption patterns and adherence to the institutional febrile neutropenia protocol in autologous stem cell transplant patients.

METHOD:

Retrospective, cross-sectional study conducted at a tertiary hospital. Included were patients undergoing autologous stem cell transplantation between September 2024 and May 2025 who developed FN, received antimicrobials, and were discharged. Data were extracted from the hospital's electronic system. Antimicrobial use was assessed via unit-level consumption reports. Protocol adherence by physicians, nurses, and pharmacists was evaluated using an institutional checklist covering blood culture collection, venous blood gas analysis, and antibiotic initiation within 60 minutes of protocol activation. The length of hospital stay was used as a clinical outcome indicator.

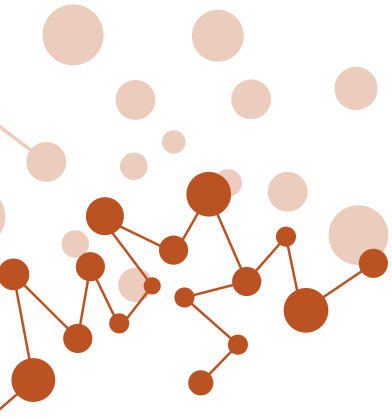
RESULTS:

Sixteen patients met the inclusion criteria; one was excluded due to death. Protocol adherence was high, with only one noncompliance related to omission of venous blood gas analysis. The average time to first antimicrobial dose was 23 minutes. Blood cultures were positive in 40% of cases, with pathogens including Klebsiella pneumoniae, Streptococcus mitis, coagulase-negative Staphylococcus spp., and Enterobacter cloacae complex. The most frequently used antimicrobials were cefepime (67%), meropenem (60%), and teicoplanin (60%), with a mean treatment duration of 6 days. The average hospital stay was 19 days, with no ICU admissions.

CONCLUSION:

High adherence to the FN protocol by the multidisciplinary team was associated with early initiation of antimicrobial therapy, potentially contributing to reduced treatment duration and favorable clinical outcomes. These findings underscore the importance of structured, team-based approaches in managing FN in BMT settings.

KEYWORDS: Bone Marrow Transplantation, Febrile Neutropenia, Antimicrobial Stewardship



PHYSIOTHERAPY

CAN HAND GRIP STRENGTH PREDICT VARIATION IN VENTILATORY PARAMETERS IN PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION?

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INTRODUCTION:

The Hematopoietic Stem Cell Transplantation (HSCT) process has been associated with prolonged periods of physical inactivity during hospitalization, leading to reduced muscle mass and aerobic capacity. Assessing the prior functionality of patients undergoing HSCT may help with prognosis and direct approaches that will reduce comorbidities and hospital stay. However, it is still unclear whether strength and ventilatory variables, essential for determining a patient's functionality, are related to each other.

OBJECTIVES:

To evaluate whether hand grip strength (HGS) can predict spirometric parameters in patients undergoing HSCT.

METHODS:

We previously assessed the functionality of patients undergoing HSCT. The HGS (in KgF) was assessed using a hand grip dynamometer. Spirometric parameters [Forced Vital Capacity (FVC) and forced expiratory volume in the first second (FEV1)] were assessed using spirometry test. All tests were performed approximately 30 days before HSCT (D-30). The differences were considered statistically significant when $P < 0.05$.

RESULTS:

We included 72 patients undergoing HSCT, of whom 40,27% (n = 29) were women. The mean age was 58.08 (SD = 9.28) years. Right-HGS is positively correlated with FVC ($r^2 = 0.3955$; $p = < 0.001$) and can explain 39,5% of the variation in total exhaled volume [Figure 1A]. In addition, right-HGS is also positively correlated with FEV1 ($r^2 = 0.3399$; $p = < 0.001$), and can predict 33,9% of the variation in volume exhaled in the first minute [Figure 1B]. We also demonstrated that left-HGS is also positively correlated with FVC ($r^2 = 0.3823$; $p = 0.0001$) and can explain 38,2% of the variation in total exhaled volume [Figure 1C]. As expected, left-HGS is positively correlated with FEV1 ($r^2 = 0.3222$; $p = < 0.001$), and can predict 32,2% of the variation in volume exhaled in the first minute [Figure 1D]. The equations of the straight lines, as well as their respective correlations, are shown in Figure 1A-D.

CONCLUSION:

Here, we demonstrated a significant association between muscle strength (measured by HGS) and respiratory capacity in patients subsequently undergoing HSCT. Thus, the assessment of muscle strength predicts, at least in part, the variation in ventilatory parameters. Furthermore, these two parameters may guide prehabilitation strategies for patients who are eligible for HSCT, improving prognosis and reducing the chances of complications during hospitalization. However, our data are still preliminary.

KEYWORDS: hematopoietic stem cell transplantation, prehabilitation, muscle strength

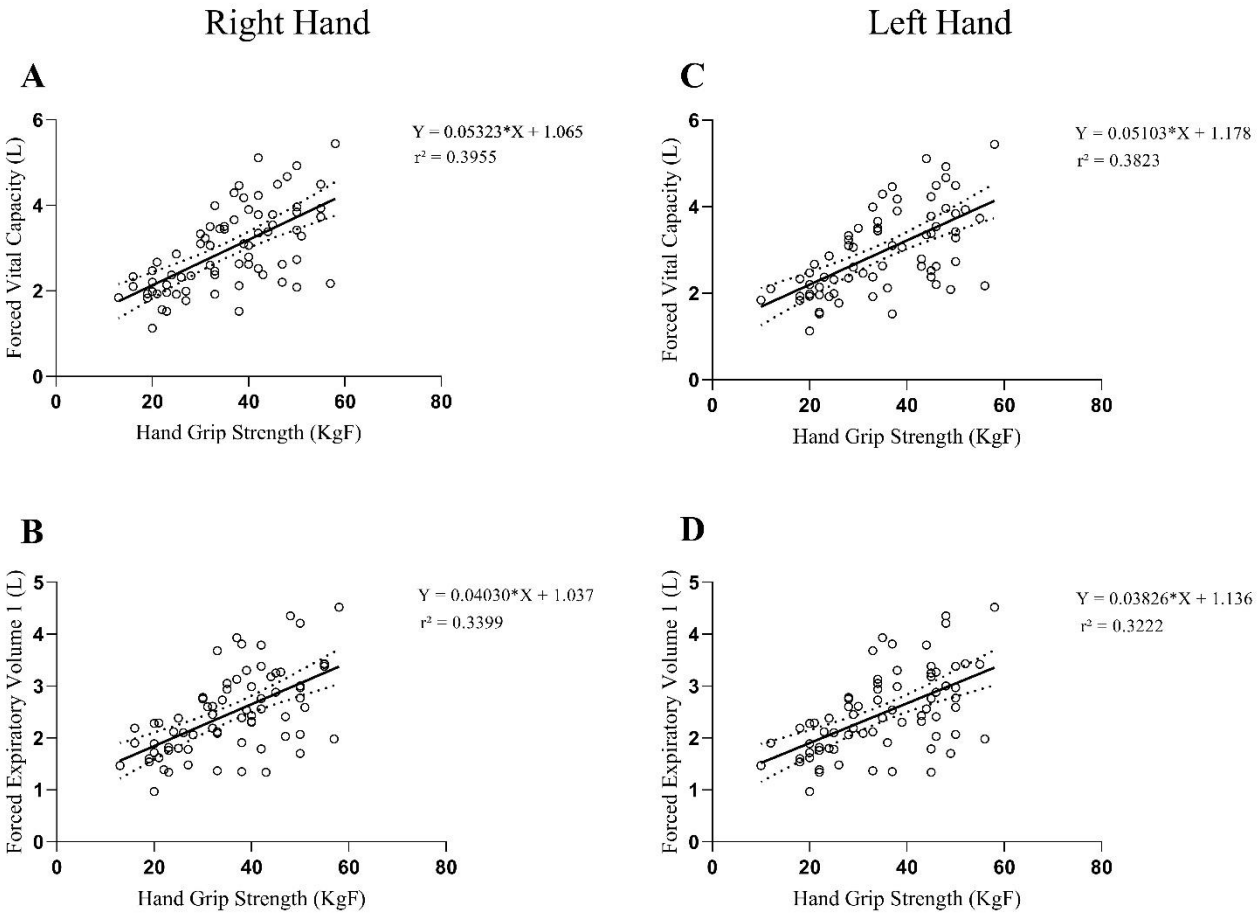

Figure 1. Right hand grip strength is moderately correlated with FVC (A) and FEV1 (B), and can predict 39,5% and 33,9% of the variation in exhaled volumes, respectively. In addition, left hand grip strength is also moderately correlated with FVC (C) and FEV1 (D), and can predict 38,2% and 32,2% of the variation in exhaled volumes, respectively.

DOES THE PRESENCE OF GRAFT-VERSUS-HOST DISEASE HAVE AN IMPACT ON THE QUALITY OF LIFE OF POST-BONE MARROW TRANSPLANT PATIENTS?

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INTRODUCTION:

Bone marrow transplantation is a complex procedure that can have an impact on patients' quality of life, especially in cases of allogeneic transplantation. One of the main complications associated with this type of transplant is Graft versus Host Disease. This condition occurs when the cells of the recipient's immune system recognize the donor's cells as antigens, resulting in various chronic damages to the body. These problems can influence the physical and emotional dimensions of individuals, making it necessary to assess the quality of life of these patients in order to understand the impacts and thus contribute to more effective care strategies in the post-transplant context.

OBJECTIVE:

To assess the impact of graft-versus-host disease on the physical quality of life of patients undergoing bone marrow transplantation.

METHOD:

This is a quantitative and descriptive field study. The research was carried out in Fortaleza, Ceará between March and May 2025. Data was collected using a sociodemographic questionnaire and the SF-12, a validated instrument that quantifies quality of life in relation to mental and physical aspects.

RESULTS:

The study included 29 patients undergoing allogeneic BMT, of whom 18 (62.1%) were diagnosed with GVHD. The predominant age group was between 40 and 60 years (48.3%) and the majority were male (65.5%). Based on the SF-12 scores, 41.4% of the patients showed impairment in physical health, 6.9% in mental health, and 31% in both domains. Only 20.7% showed no significant changes. These data indicate that the majority of patients with Graft Disease have some impact on their quality of life, mainly in physical aspects, which may be related to the acute and chronic manifestations of the disease, such as pain, respiratory fatigue, skin lesions and functional limitations. The impact on mental health, although less frequent in isolation, becomes more expressive when associated with physical complaints, which suggests an influence between the domains.

CONCLUSION:

The results of this study show that Graft-versus-Host Disease in patients undergoing allogeneic bone marrow transplantation has an impact on quality of life, with greater impairment in the physical dimensions. Furthermore, the association between physical and mental alterations reinforces the need for multi-professional follow-up and regular assessment to promote more assertive post-transplant care.

PHYSIOTHERAPEUTIC MANAGEMENT IN A PATIENT WITH REFRACTORY MYASTHENIA GRAVIS UNDERGOING AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION: A CASE REPORT

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INTRODUCTION:

Myasthenia Gravis (MG) is an autoimmune disease that affects the neuromuscular junction, causing fluctuating muscle weakness. In refractory cases—especially after infectious events such as COVID-19—clinical progression may require advanced immunomodulatory therapies. Due to refractoriness to standard clinical treatment, the patient was referred for evaluation and inclusion in a protocol for autologous hematopoietic stem cell transplantation (TCTH), an approach still restricted to severe cases unresponsive to conventional therapies.

OBJECTIVES:

To report the physiotherapeutic management in the first known case of TCTH in a patient with refractory MG in Brazil, with emphasis on respiratory and functional interventions.

METHODS:

Daily physiotherapy sessions were performed according to the patient's clinical conditions. The therapeutic resources used were non-invasive ventilation, respiratory kinesiotherapy, motor exercises with dumbbells and ankle weights on alternate days, TENS at specific points for pain control, thermotherapy, lymphatic drainage, and cycle ergometer.

RESULTS:

The physiotherapy team played a crucial role in the patient's care, using a variety of approaches to optimize respiratory function, muscle strength, and quality of life. Interventions were aimed at preserving muscle strength, improving joint mobility, reducing fatigue, and enhancing respiratory capacity. Despite the challenges faced, the patient experienced significant benefits with physiotherapy support. Through an integrated, patient-centered approach, it was possible to minimize the adverse effects of MG treatment and, during hospitalization, promote a more positive experience for the patient.

CONCLUSION:

physiotherapeutic care in a patient with refractory MG undergoing TCTH was essential for maintaining respiratory function and muscle strength, contributing to a safe and functional clinical recovery. This case highlights the importance of an interdisciplinary and individualized approach in complex cases.

KEYWORDS: Myasthenia Gravis, Stem Cell Transplantation, Physiotherapy, Rehabilitation, Autoimmune Disease

RESPONSE OF ACTIVE RESISTANCE MOTOR KINESIOTHERAPY IN THE EVALUATION OF FALL RISK PREVENTION ON THE DEGREE OF MUSCLE STRENGTH AND MOBILITY USING THE FSS, MRC AND TUG FUNCTIONAL TESTS IN PATIENTS UNDERGOING BONE MARROW TRANSPLANTATION

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INTRODUCTION:

The prevention of falls in patients undergoing bone marrow transplantation (BMT) is of great importance because falls represent a significant risk to the health of individuals in this population. Falls during hospitalization are related to pathophysiological factors such as anaemia and fatigue, as well as the effects of different treatments such as chemotherapy and radiotherapy, which lead to diarrhoea, vomiting, dizziness, muscle weakness, reduced and/or limited mobility and the frequent changes in the clinical picture that characterize these individuals during hospitalization, in addition to the manifestations of the disease itself. Physiotherapy assessment seeks to identify individual risk factors, such as mobility deficits and muscle weakness.

OBJECTIVE:

To assess the extent to which active resistance motor kinesiotherapy prevents the risk of falls in patients undergoing bone marrow transplantation through functional tests on the degree of muscle strength and mobility.

METHOD:

This study followed a longitudinal protocol. Ten individuals took part in the study and underwent functional tests such as the FSS, MRC and TUG before and after BMT, as well as physiotherapeutic intervention with active resistance motor kinesiotherapy before and after BMT. Active resistance motor kinesiotherapy was applied on the first day of hospitalization, as well as the functional tests, and continued until discharge. The patients underwent daily active resistance motor kinesiotherapy for 16 days using an elastic band, ball, 1 kg shin guards and 1 kg dumbbells. The degree of muscle strength was measured using the MRC, mobility was assessed using the FSS scale and the risk of falling was assessed using the TUG. The Student's t-test was used for statistical analysis.

RESULTS:

Participants (n = 10 men n = 7) transplanted (autologous n = 6 allogeneic n = 4) men aged 51.9 ± 8.1. Aplasia time 11.3 ± 1.1 days. MRC at admission 42.4 ± 1.3 and at hospital discharge 50.1 ± 6.8 and $p = 0.001$. TUG at admission 12.1 ± 1.9 and at discharge 7.2 ± 1.9 and $p < 0.05$.

CONCLUSION:

This study revealed the importance of the use of active resistance motor kinesiotherapy in gaining muscle strength and improving the mobility of patients undergoing BMT. It also showed the important use of functional tests in assessing these patients. However, more studies on the subject are needed to determine the magnitude of these findings.

THE IMPACT OF PHYSICAL THERAPY ON FUNCTIONAL RECOVERY IN BONE MARROW TRANSPLANT PATIENTS: A SAFETY AND EFFICACY STUDY

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BACKGROUND:

Bone marrow transplant (BMT) patients are highly susceptible to functional decline and physical deconditioning due to prolonged hospitalization, immunosuppression, and the impact of hematological diseases. Physical therapy (PT) during the BMT process aims to mitigate these effects, enhancing functional recovery and overall prognosis. However, evidence on the effectiveness and safety of PT interventions in this population remains limited, particularly in real-world hospital settings.

OBJECTIVE:

This study aimed to assess the safety and efficacy of a physical therapy program in BMT patients, comparing pre- (admission) and post- (discharge) outcomes.

METHODS:

This observational study included BMT patients who adhered to the PT program (adherence >70%). Data were collected at hospital admission (pre) and discharge (post) BTM. The primary outcomes included functional capacity (Step Test performance), muscle strength (Handgrip Test), and fatigue (BFI - Brief Fatigue Inventory). Safety was evaluated through the incidence of PT-related complications, including cardiorespiratory or hospitalization adverse events. Mortality and length of hospital stay were also recorded.

RESULTS:

Among the 12 adherent patients, the mean age was 47±19 years, with 60% females. The Step Test performance showed a slight reduction from pre to post (mean difference: -18±25 steps, p=0.21). Handgrip strength remained stable between admission and discharge (mean difference: -0.5±3 kg, p=0.94). Fatigue scores showed a minor increase (BFI: pre 0.96±0.82, post 1.11±1.07), but remained within acceptable limits. Pulmonary function was preserved (92.8% VEF1), and mobility scores showed improvement (JHM: 5.7±2.7 to 7.0±2.0), while the Karnofsky Performance Scale (KPS) remained stable (85±20 to 80±12, p=0.75). No adverse events related to physical therapy were recorded. The overall mortality rate among adherent patients was 8%, and the mean length of stay was 34±11 days.

CONCLUSION:

The results indicate that adherence to physical therapy during bone marrow transplantation may play a protective role in maintaining functional capacity and muscle strength, despite the physical burden of the treatment. The stable mobility and muscle strength observed, along with the minimal increase in fatigue levels, suggest that rehabilitation protocols can be safely integrated into patient care without compromising outcomes. These findings reinforce the importance of structured physical therapy programs to support functional preservation during hospitalization.

UNDERSTANDING HAND GRIP STRENGTH AS A PREDICTOR OF FUNCTIONAL CAPACITY: A LINEAR REGRESSION ANALYSIS IN PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) requires protective isolation, causes numerous adverse effects and prolonged physical inactivity. Assessing the functional capacity and prior muscle strength of patients undergoing HSCT may guide approaches capable of improving prognosis and reducing hospital stay. However, it is unclear whether any of these variables acts as a predictor of another and whether there is a correlation between them.

OBJECTIVES:

To verify whether handgrip strength (HGS) can predict functional capacity in patients undergoing autologous HSCT.

METHODS:

This observational cross-sectional study included patients over 18 years of age undergoing autologous HSCT. Handgrip strength was assessed by kilogram-force (kgF) on an analog dynamometer and functional capacity was quantified by the distance covered in the 6-minute walk test (6MWT). Data collections were made in the pre-HSCT period (D-30). Associations were calculated by simple linear regression and the significance level adopted was $p<0.05$.

RESULTS:

62 patients were evaluated, 58.06% (n=36) male, 58.92 ± 9.61 years old. The mean right HGS was 36.14 ± 12.61 kgF and the left HGS was 35.22 ± 12.48 kgF. The mean distance covered in the 6MWT was 460.8 ± 107.1 meters, 45.16% did not reach the predicted distance (517.5 ± 66.8 meters). A moderate and statistically significant positive correlation was observed between the 6MWT and the right HGS (r^2=0.397 | p=0.0001 | 95% CI 3.648–7.054), indicating that 39.7% of the variation in the distance covered in the 6MWT can be predicted by the right HGS. The equation of the estimated straight line (Y=5.35*X+237.4) suggests that for each increase of 1 kgF in the HGS there was an average increase of 5.35 meters in the 6MWT. In relation to the left HGS and the 6MWT, a moderate positive and statistically significant correlation was found (r^2=0.347 | p=0.0001 | 95% CI 3.269-6.849), 34.7% of the variability in the 6MWT is predicted by the left HGS. According to the equation of the estimated straight line (Y=5.05*X+252.6) for each 1 kgF there was an average increase of 5.05 meters in the 6MWT.

CONCLUSION:

Simple linear regression analysis demonstrated a significant correlation between handgrip strength and functional capacity measured by the distance covered in the 6MWT in patients in the pre-autologous HSCT period (D-30). Therefore, HGS can be a predictor and marker of functional capacity. In this sense, prehabilitation strategies for patients eligible for transplantation, focused on the strength variable, can have a functional impact, reducing hospital stay, improving performance and prognosis. New studies with a large sample are needed to improve the reliability of the findings.

KEYWORDS: Hematopoietic stem cell transplantation, Functional capacity, Muscle Strength.

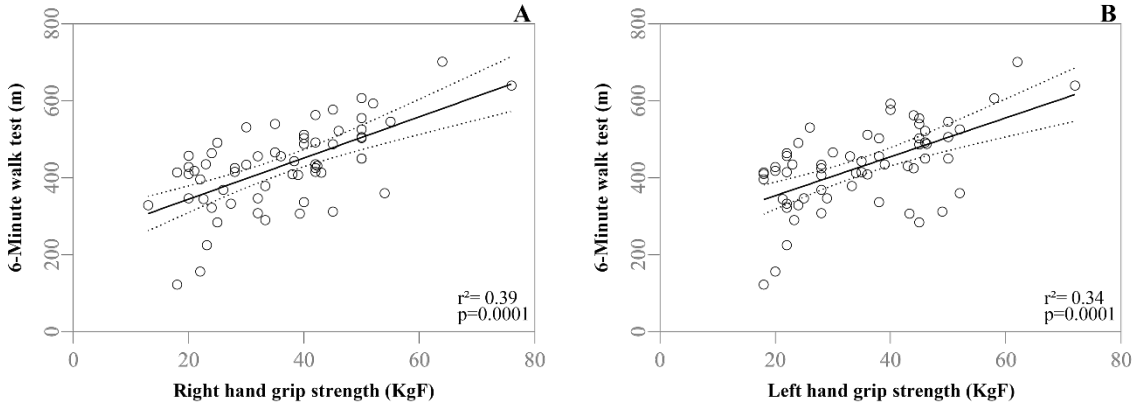
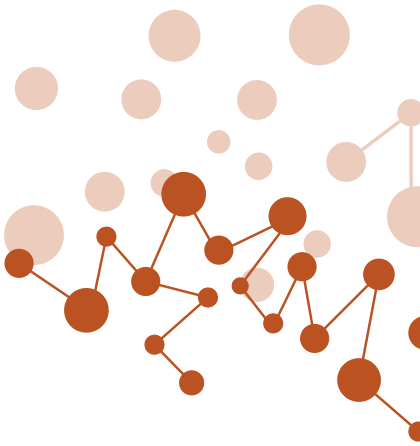


Figure 1: (A) Positive and moderate correlation between right HGS and 6MWT, 39% of the distance covered in the 6MWT is predicted by the right HGS. **(B)** Positive and moderate correlation between left HGS and 6MWT, 34% of the distance covered in the 6MWT is predicted by the left HGS.

NUTRITION



DESCRIPTIVE AND CORRELATIONAL ANALYSIS OF THE NUTRITIONAL PROFILE OF ADULTS UNDERGOING AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a treatment used to treat severe hematologic diseases and some types of neoplasms. The nutritional status of patients undergoing HSCT can influence the quality of life, prognosis, and tolerance of the proposed treatment.

OBJECTIVE:

To describe the nutritional profile of adults undergoing autologous HSCT and to analyze the correlation of nutritional measurements.

METHODS:

This cross-sectional observational study included adult patients aged between 18 to 60 years who underwent autologous HSCT and were evaluated in the pre-transplant period. Nutritional status was determined by mid arm circumference (MAC) in centimeters (cm), by the Patient-Generated Subjective Global Nutritional Assessment (PG-SGA), and by the Body Mass Index (BMI) in kilograms per square meter (kg/m^2). Data were expressed as means and proportions. The normality test used was the Shapiro-Wilk test and correlational analysis was calculated using Pearson's coefficient, with a significance level of $p<0.05$.

RESULTS:

36 patients were evaluated, 66.6% men and 33.3% women. The mean age was 51.56 ± 7.34 years. Of these, 91.6% (N=33) were diagnosed with multiple myeloma and 8.3% (N=3) with non-Hodgkin's lymphoma. According to the PG-SGA, 83.3% of patients were classified as eutrophic, while according to the MAC, 72.2% were eutrophic. The BMI calculation showed that 47% were obese, of which 53% were grade I, 29.4% grade II and 17.6% grade III. Figure 1 demonstrates a strong positive correlation between BMI and MAC with Pearson's R of |0.85| (95% CI 0.73-0.92) and a statistically significant difference (p=0.0001). During hospitalization, 83.3% of patients required oral nutritional therapy due to side effects of conditioning chemotherapy and enteral nutritional therapy was used in only 1 patient. The mean length of hospital stay was 17.8 ± 3.58 days and during this period the mean percentage of weight loss was 4.72%.

CONCLUSION:

Considering the above, most patients presented adequate nutritional status according to MAC and PG-SGA, BMI denoted that almost half of the sample had some degree of obesity. There was a correlation between BMI and MAC, that is, patients with higher BMI tend to have higher MAC. This relationship reinforces the importance of using anthropometric measurements as auxiliary tools in nutritional risk screening. Furthermore, the need for nutritional monitoring is clear, aiming at the maintenance and recovery of nutritional status.

KEYWORDS:

Hematopoietic Stem Cell Transplantation. Nutritional Assessment. Nutritional Status.

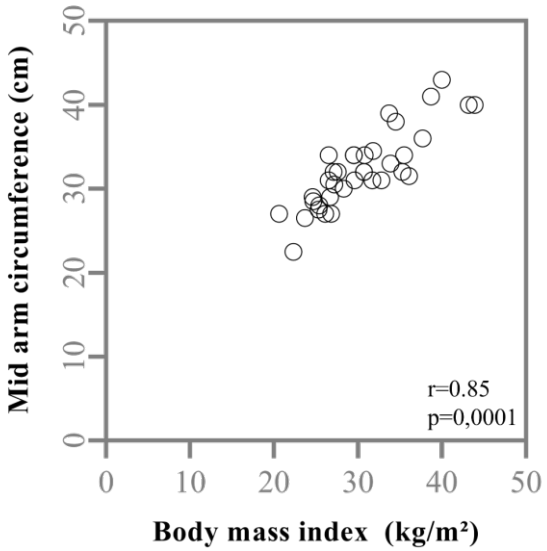
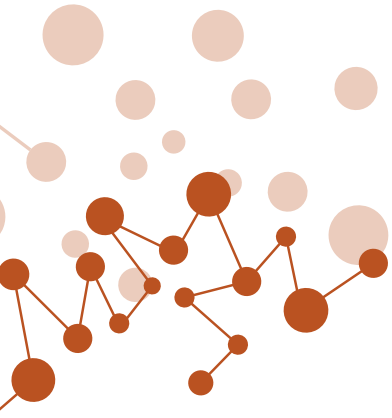


Figure 1: Pearson correlation coefficient between BMI and MAC. Pearson's R of |0.85| demonstrating a strong and positive correlation with a statistically significant difference (p=0.0001).



ODONTOLOGY

DENTAL TRETAMENT NEEDS IN HEMATOPOIETIC STEM CELL TRANSPLANTATION CANDIDATES AT TWO PRIVATE HOSPITALS IN BRAZIL: PRELIMINARY DATA

Patrícia Maria Fernandes[1,2]; Renata Belarmino[1,2]; Daniel Campanhã[1]; Roberto Luiz da Silva[1,2]; Maria Cristina Martins de Almeida Macedo[1,2]; Sumatra Melo da Costa Pereira Jales[1,2]

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INTRODUCTION:

The identification of active or potential sources of infection in the oral cavity is a crucial step in preparing patients for hematopoietic stem cell transplantation (HSCT). This measure helps reduce the risk of oral infections that may negatively impact the patient's systemic health, particularly when the patient is immunocompromised. Therefore, understanding the dental profile, as well as the dental treatments needed for HSCT candidates, is essential.

OBJECTIVE:

To identify the pattern of oral hygiene, as well as the need for elective and urgent dental treatment in candidates for HSCT from two private hospitals in Brazil.

METHOD:

This retrospective, descriptive, cross-sectional, quantitative preliminary study analyzed data from patients who were candidates for autologous or allogeneic HSCT at two private centers in Brazil between January and December 2024. Oral hygiene status was evaluated based on the presence of biofilm, dental calculus, gingivitis, and tongue coating and classified as satisfactory, intermediate, or unsatisfactory based on clinical findings. Dental treatment needs were identified and categorized as elective or urgent. Data were analyzed with descriptive statistics (frequency and percent). The study is approved by the ethics committee under CAAE 79010124.9.1001.0072.

RESULTS:

Data from a total of 53 patients were included in this study. Twenty-three (43.4%) required dental procedures. Of these, 20 patients (86.6%) needed elective dental treatment, while 3 patients (13.0%) required urgent dental treatment. Regarding oral hygiene status, most patients (92.4%) presented satisfactory oral hygiene, while 7.5% exhibited intermediate hygiene.

CONCLUSION:

The results indicate that most patients required oral dental care prior to undergoing HSCT. Oral health adequacy as well as oral hygiene guidance plays a key role in preventing local and systemic infections during the immunosuppression period, highlighting the importance of including dentists as integral members of HSCT teams.

KEYWORDS: hematopoietic stem cell transplantation; oral dental care; oral health.

THE ROLE OF MULTIDISCIPLINARY DENTAL CARE IN REDUCING ORAL MUCOSITIS IN AUTOLOGOUS BONE MARROW TRANSPLANT PATIENTS: PRELIMINARY FINDINGS

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INTRODUCTION:

Autologous Bone Marrow Transplantation (ABMT) is a therapeutic procedure indicated for various hematological diseases, such as lymphomas and multiple myeloma. Its success depends, among other factors, on the proper management of complications associated with the patients' immunosuppressed state until engraftment occurs. Oral mucositis is one of the most prevalent and debilitating oral complications faced by the transplant team.

OBJECTIVE:

This study aims to present an epidemiological assessment of the first 16 cases of individuals undergoing ABMT in a specialized unit, discussing the impact of a pre-transplant oral care protocol, including oral environment preparation, biofilm control, and daily laser photobiomodulation, on the prevention of oral mucositis.

METHOD:

Sixteen patients undergoing autologous BMT in a hospital setting were followed. All patients received a pre-conditioning dental evaluation and intervention, including removal and control of infectious foci, oral hygiene guidance, and daily low-level laser photobiomodulation sessions throughout the period of immunosuppression.

RESULTS:

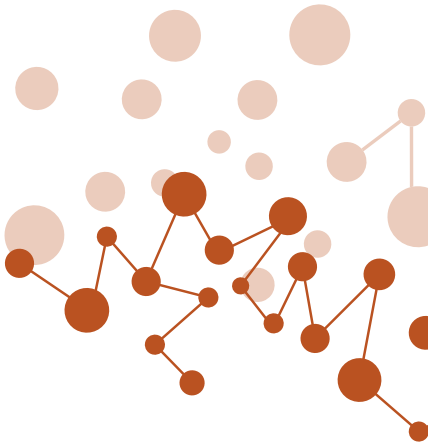
During the follow-up, none of the patients developed oral mucositis ($p < 0.001$), one of the most expected side effects, demonstrating that the dental care protocol adopted by the team was successful. There were reports of nausea and dysgeusia, which are expected symptoms in the context of chemotherapy, but these did not progress to oral complications. Patient adherence to dental guidelines was satisfactory in all cases, supporting the maintenance of oral health during the transplant period.

CONCLUSION:

The presence of the dental team in the multidisciplinary care of patients undergoing ABMT proved to be fundamental in preventing oral complications, especially mucositis, contributing to safer treatment, with lower morbidity and better quality of life. The combination of daily photobiomodulation, biofilm control, and prior dental intervention proved to be highly effective and should be considered an integral part of BMT protocols. In this context, the role of Dentistry in the preparation and multidisciplinary follow-up of transplant patients is essential for preventing complications, promoting oral health, and improving the quality of life of the transplanted patient.

KEYWORDS: Bone Marrow Transplantation, Stomatitis, Preventive Dentistry.

PSICOLOGY



CLINICAL LISTENING AND PSYCHOLOGICAL MONITORING IN BONE MARROW TRANSPLANTATION: EXPERIENCE REPORT

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Bone Marrow Transplantation (BMT) is a therapy for refractory neoplasms and hematologic diseases. From diagnosis to post-transplant, patients and their caregivers face intense emotional experiences. Studies in the area show that the psychological distress of both is influenced by the conditions of hospitalization, the withdrawal from social life and the wait for therapeutic results, which can trigger anxiety, irritability, depression and lack of motivation. Thus, the work of the hospital psychologist, in conjunction with the multidisciplinary team, is necessary. Through clinical listening, the psychologist addresses emotional demands, helps to strengthen resilience and contributes to the management of expectations throughout the treatment.

OBJECTIVE:

To report the experience of the psychology service in a BMT unit, focusing on clinical listening as the axis of monitoring patients and family members.

EXPERIENCE REPORT:

Psychological monitoring begins before the transplant, with a structured assessment of the patient and family. The psychosocial structure, coping resources, possible psychiatric disorders, understanding and desire for treatment, and the support network are investigated. During hospitalization, bedside care is provided according to the needs of each case. The action aims to sustain psychological integrity in the face of uncertainty, vulnerability, and isolation. The main demands include fear of adverse effects, anxiety about the bone marrow "taking", loneliness, and clinical unpredictability. Phrases such as "we knew it would be difficult, but it is beyond what we imagined" are frequent and express the mismatch between imagination and reality. Clinical listening, combined with psychoeducational interventions, offers support and favors emotional processing. These strategies promote expression, validation, and resignification of the experience, allowing psychological reorganization and the search for meaning. Post-transplantation, challenges persist and impact quality of life due to physical limitations, changes in body image, loss of social roles and fear of recurrence. The service offers outpatient monitoring, based on spontaneous demand, in order to support reintegration into daily life and coping with new conditions.

CONSIDERATIONS:

The experience in TMO highlights the relevance of psychological work for comprehensive care, valuing the uniqueness of each trajectory. However, challenges remain, such as expanding the team's understanding of subjective demands, making practices more flexible, and recognizing the importance of psychological assessments. The availability of psychiatric support for intense emotional crises is also essential. These aspects point to the need for person-centered care that integrates technique, sensitivity, and support.

KEYWORDS:

Bone Marrow Transplantation, Psychological Support, Clinical Listening

THE ENTRY POINT TO PSYCHOLOGICAL CARE: THE PRE-TRANSPLANT INTERVIEW AS THE FIRST THERAPEUTIC TOOL

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INTRODUCTION:

Bone Marrow Transplantation (BMT) is the proposed treatment in cases of onco-hematological or hematological diseases that do not respond satisfactorily to more conservative approaches. As such, most patients referred for this type of care arrive carrying the emotional impacts of the diagnosis and prior interventions from their original treatment institutions. These previous experiences influence how the patient approaches the first contact with the team responsible for the BMT and may provide indications of how their journey will unfold. Upon arrival at the transplant institution, the patient undergoes a medical consultation to confirm the indication for the procedure. If deemed eligible, the patient is referred for screening by the multidisciplinary team. For Psychology, the pre-transplant interview is conducted through the Psychological Anamnesis—a semi-structured framework aimed not only at understanding the patient's psychic, familial, and social dynamics, and assessing their level of engagement with the illness and treatment, but also at identifying their coping resources.

OBJECTIVE:

To present the pre-transplant interview as a tool for building a therapeutic bond and developing diagnostic hypotheses.

METHODOLOGY:

Experience report based on the work of the psychologist at the pre-bone marrow transplant clinic at a university hospital between 2014 and 2024. The public is made up of adult and elderly patients with an indication for the procedure and their companions. The work is guided by psychoanalytic theory and the techniques used are psychological welcoming, crisis intervention and individual or family care. The instrument used is the Psychological Anamnesis.

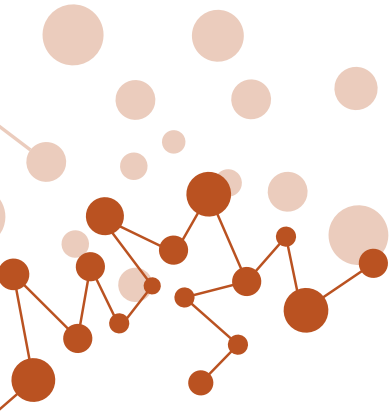
RESULTS:

The pre-transplant psychological interview is seen as the patient's main entry point to care in the sector. In this first contact, the patient is given a Psychological Anamnesis, a semi-structured script that covers data on the family configuration and experiences from the patient's childhood to adulthood, social ties with work and the community, circumstances of the diagnosis and health treatments carried out so far, perceptions and expectations regarding the proposed transplant. As well as fostering the therapeutic bond, the pre-transplant interview provides a broad view of the patient's psychic and social dynamics, helping to identify early on those patients who will need greater emotional support during the process and providing elements that can be managed together with the other professionals on the team in order to minimize the risk to the patient.

CONCLUSION:

The pre-transplant psychological interview does not have the prerogative of indicating the transplant or not, but it is an important tool for building the therapeutic bond and preventing possible psychological complications during the process.

KEYWORDS: Psychological anamnesis; Bone marrow transplantation; Psychoanalysis.



SOCIAL SERVICE

THE RELEVANCE OF WELCOMING AND HUMANIZATION IN SOCIAL WORK CARE IN PEDIATRIC BONE MARROW TRANSPLANTATION UNDER PROADI-SUS

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The relevance of welcoming and humanization in Social Work care in the context of pediatric Bone Marrow Transplantation (BMT) is central and multifaceted, as it involves care for both the child undergoing treatment and their family. BMT is a complex, painful, and prolonged process that provokes fear, insecurity, and emotional suffering. Qualified welcoming enables the Social Work team to identify these anxieties and act to mitigate emotional and social impacts by providing continuous psychosocial support. Humanization recognizes the child as a subject of rights, valuing their history, expression, and experience during treatment. This also involves promoting a more welcoming, playful, and understanding environment tailored to the child's needs. Children who feel safe and welcomed, along with families who are well guided and supported, tend to cope better with the challenges of BMT. To analyze the relevance of welcoming and humanization in the care provided by the Social Work team in the context of pediatric BMT, demonstrating how these principles contribute to comprehensive, ethical, and effective care that includes both the child in treatment and their family—strengthening bonds, ensuring rights, and contributing to better clinical outcomes. This is a cross-sectional study with data collected from June 2024 to April 2025, involving adolescent and child patients of both sexes, aged 2 to 15 years. The study includes both quantitative and qualitative components, as it examined aspects related to the use of support houses and the behavior of patients and families during hospitalization in the pre- and post-BMT periods under the PROADI-SUS program. Social Work consultations were conducted both remotely via telemedicine and in person with patients and their families. Of the 11 patients studied, 6 (54.5%) were female. Ten patients (90.9%) used accommodation in support houses. The average length of stay in São Paulo ranged from 2 to 4 months. Three patients (27.3%) were from the state of São Paulo; one (9.1%) from Rio de Janeiro; one (9.1%) from Minas Gerais; and six (54.5%) from Espírito Santo. We found that patients and families accommodated in support houses received differentiated welcoming, including emotional support from a psychology team available as needed. Comprehensive support in the form of meals, transportation, and occupational therapy—extending beyond the hospital setting—helped promote care in its broadest sense: physical, emotional, subjective, and social. An approach based on humanization and qualified welcoming strengthens the quality of care provided in pediatric BMT, enabling better treatment outcomes, relief of suffering, and the promotion of human dignity. More than institutional practices, these principles serve as ethical and political foundations guiding care that is committed to the full protection of childhood and the centrality of the individual in the care process.

KEYWORDS: Bmt, Sus, Proadi, Bone Marrow Transplantation

THE ROLE OF INSTITUTIONAL SHELTER SERVICES FOR PATIENTS UNDERGOING HIGH-COMPLEXITY TREATMENT AWAY FROM THEIR PLACE OF RESIDENCE

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INTRODUCTION:

High-complexity treatments, such as bone marrow transplants, are concentrated in large urban centers, requiring patients to travel for appropriate care. The Out-of-Home Treatment (TFD) program is a public policy designed to ensure this access; however, it faces financial and operational limitations. In this context, institutional shelter services play a fundamental role by providing free accommodation and comprehensive support to vulnerable patients, promoting continuity of treatment and the realization of the right to health.

OBJECTIVE:

To demonstrate the importance of an institutional shelter service for patients undergoing treatment for severe diseases away from their home.

METHOD:

This study adopts a descriptive approach to outline the functions and activities of an institutional shelter service for patients and their families who travel to southern Brazil in search of high-complexity treatments.

RESULTS:

The service is provided in an institutional unit with home-like characteristics, aiming to meet the needs of the individuals being hosted, ensuring adequate conditions of dignity, hygiene, accessibility, privacy, security, and comfort, as well as ensuring full access to rights and public services. The target audience consists of individuals aged 16 or older, undergoing treatment away from their home due to oncological-hematological diseases, particularly those undergoing bone marrow transplants, referred both by public network services and through spontaneous demand. The technical team carries out various interventions, focusing on reception and qualified listening, referral to public services and bodies responsible for defending rights, as well as developing actions aimed at promoting coexistence, adherence to treatment, autonomy, and self-care through discussion circles, informational lectures, and thematic workshops.

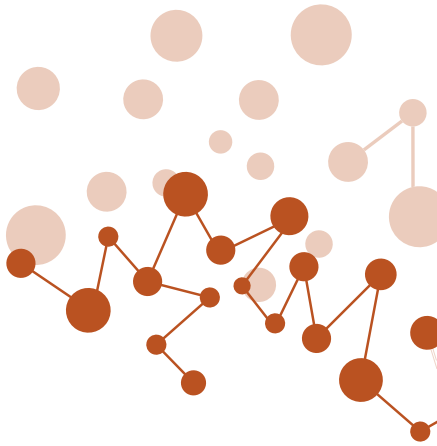
CONCLUSION:

This study highlighted the importance of institutional shelter services in promoting continuous access to high-complexity treatments, contributing to the well-being of patients and their caregivers during a period of vulnerability. The findings emphasize the integration of social assistance and healthcare as a crucial element in ensuring the right to health. Future studies should investigate the long-term effects of these services on patient recovery and identify strategies to increase funding and institutionalize these initiatives within the public health system.

KEYWORDS:

Out-of-Home Treatment-TFD, Institutional Shelter, Bone Marrow Transplant.

OTHERS



A VIEW FROM NURSING AND NUTRITION: AS A STRATEGY OF CARE AND SAFETY IN HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION

Hematopoietic Cell Transplantation (HSCT) is a procedure that involves high risks and rigorous care, from patient preparation to post-transplantation. Nursing and Nutrition occupy central roles in care, continuous monitoring and the promotion of patient safety.

OBJECTIVE

Discuss the importance of joint action between Nursing and Nutrition professionals as fundamental care and safety strategies during all stages of HSCT.

METHOD

This study is characterized as a descriptive research, with a qualitative approach, with the objective of understanding and analyzing the care and safety strategies adopted by the Nursing and Nutrition teams during the hematopoietic stem cell transplantation (HSCT) process. The choice of this approach is justified by the need to explore perceptions, practices and experiences of the professionals involved, as well as to highlight their contribution to the quality of care and patient safety.

RESULTS

Nursing is responsible for applying care protocols, administering therapies with precision, monitoring clinical signs, and intervening in adverse situations. The implementation of safety checklists has proven to be an effective tool in the prevention of errors and adverse events, ensuring the standardization of conducts and continuity of care. In addition, nursing works in the education of patients and families, promoting self-care and adherence to treatment. Immunosuppressive therapy brings complications that influence nutritional status. Reactions such as vomiting, nausea, diarrhea, mucositis, hyporexia, or anorexia are common. Early and regular nutritional assessment with patients who will undergo HSCT should be part of a protocol. Interventions help to improve nutritional status, control symptoms. Daily nutritional adjustments, continuous monitoring, help in the recovery of nutritional status. Effective communication among the multidisciplinary team is essential for the success of HSCT and for improving the quality of life of patients during and after treatment, contributing to comprehensive care, promoting better clinical outcomes. The joint action strengthens the bond with the patient, who feels more welcomed and safe.

CONCLUSION

The integration between Nursing and Nutrition is an essential strategy for the safety of patients undergoing HSCT. Multiprofessional care, based on evidence and well-defined protocols, contributes to risk reduction, improves the quality of care, and favors patient recovery.

KEYWORDS:

Hematopoietic Cell Transplantation (HSCT), Nursing, Nutrition.

CHALLENGES FOR TEACHERS IN TEACHING IN THE HEMATOPOIETIC STEM CELL TRANSPLANT SECTOR - HSCT

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INTRODUCTION:

The project Multidisciplinary work, sociodemographic and emotional profile and school performance: qualitative and quantitative assessment in an Onco-hematology service highlights the importance of multidisciplinary work in highly complex treatments and presents the challenges of teaching work at the interface between health and education (Hospital Schooling Program SME/CURITIBA; Hospital Schooling Network Support Service SAREH/SEED/PR).

OBJECTIVE:

To expose the challenges of teaching in a highly complex sector in guaranteeing the right to education of student-patients, in interface with the health team.

CASE SERIES:

Onco-hematology and Hematopoietic Stem Cell Transplant (HSCT) patients. Sampling: 120 student-patients (March/2021 to May/2025). Median age: 7.1 years (Preschool; Elementary School I), 14.3 years (Elementary School II; High School). Main diagnoses: oncological diseases; rare diseases.

METHOD:

Descriptive-explanatory qualitative-quantitative approach, with participant intervention research design. Results: Highlights the importance of having teachers working in hospitals to ensure continuity of studies, recognizing the essential importance of education for social inclusion and the relevance of preparing school teams for the in-person return of student-patients to schools. Challenges: flow of students, who require individual care and personalized pedagogical work to fill learning gaps (common to ongoing treatments); oncological diagnoses associated with neurological conditions; expenses with materials (continuous exchange with the need for laminating for sanitation); exchange with distant schools; lack of knowledge of hospital education as a right; reduced number of teachers in pediatric hospitals; low level of education of those responsible for academic support to their children; weakened bond with learning/school, resistance to joining classes when the treatment is complex or the prognosis is unfavorable. Strengths: having its own education and culture department; welcoming and coordination between teachers and health teams; weekly multidisciplinary meetings to discuss cases and collectively build work plans. The multidisciplinary work favored personalized action that was attentive to the needs of each student-patient, so that the teaching addressed health issues that contributed to the success of the treatment (nutrition, sun exposure, oral and hand hygiene, etc.) and produced significant learning.

CONCLUSIONS:

There is a need to increase the number of education programs in Brazilian pediatric hospitals, strengthening partnerships with health teams and education systems, and increasing the number of active teachers to adequately meet the demands of each hospital/clinic.

KEYWORDS: Multidisciplinary Work – Research-Intervention – Hospital Education.

EQUITY IN ACCESS TO HEMATOPOIETIC STEM CELL TRANSPLANTATION: A NATIONAL OVERVIEW OF REFERRALS FOR TRANSPLANTATION THROUGH THE UNIFIED HEALTH SYSTEM

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INTRODUCTION:

The National Transplant System is responsible for coordinating all transplant procedures in Brazil, making it the largest public transplant system in the world, with most of its funding coming from the Unified Health System. However, a persistent issue is the centralization of hematopoietic stem cell transplant (HSCT) capacity in certain regions of the country. According to data from the National Registry of Health Establishments (NRHE), the majority of the 262 registered transplant centers are in the Southeast region, and about 40% of these belong to private institutions.

OBJECTIVE: To evaluate the geographic distribution of transplant centers and the need for referrals to other regions of Brazil.

METHOD: Descriptive, longitudinal, retrospective study conducted with hospitalization data from 2021 to 2024, using NRHE data.

RESULTS:

A total of 11,000 hospitalizations for HSCT were recorded in Brazil. Regarding the distribution of health centers, 39 are in the Southeast region, 11 in the South, 9 in the Northeast, 5 in the Central-West, and 1 in the North. The types of transplants also vary between regions. In the Southeast, 20 centers performing related donor HSCTs and 17 performing unrelated donor. In the South, 8 centers for related donors and 6 for unrelated donors. In the Northeast, there are 4 centers of each type. In the Central-West, 2 centers perform related donor HSCTs and 1 unrelated. In the North, only autologous transplants are performed. When analyzing access inequality to treatment, in the Southeast region, patients travel 125 kilometers (km) from their hometowns to the transplant center. In the South, patients travel about 130 km. In the Central-West, this average rises to 386 km. In the Northeast, it reaches around 539 km, and in the North, patients travel over 2,000 km to undergo transplantation.

CONCLUSION:

The data indicate that access to HSCT treatment is unequal across the regions of the country, being more difficult and requiring longer travel in the most critical areas, especially considering the complexity of the type of transplant needed. The National Policy for Specialized Health Care aims to expand and guarantee the population's access to specialized services in a timely manner, with territorial reference and considering regional needs, ensuring equity in care, quality of assistance, comprehensiveness, and greater effectiveness and efficiency in the use of financial resources. To improve the HSCT program, expand and strengthen access to this treatment, a health institution in the city of São Paulo proposed a project within the scope of the Institutional Development Support Program of the Unified Health System, promoting the expansion of the supply of specialized care services with a view to qualifying access, reducing regional inequalities, identifying bottlenecks in the health system and assisting in public policies that promote greater equity in access to HSCT throughout the national territory.

KEYWORDS: hematopoietic stem cell transplant, Unified Health System, equity

EXPLORING PREDICTIVE BIOMARKERS IN PEDIATRIC ACUTE MYELOID LEUKEMIA: ADVANCES IN DIAGNOSIS AND MINIMAL RESIDUAL DISEASE MONITORING

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INTRODUCTION:

Pediatric Acute Myeloid Leukaemia (AML) is a rare and aggressive disease characterized by several genetic alterations, which significantly influence the prognosis of the disease and treatment outcomes. Despite advances in treatment, pediatric AML remains a challenging condition, particularly in resource-limited settings, where access to specialized care can have a significant impact on survival rates. In this context, the identification and monitoring of Minimal Residual Disease (MRD) has become an integral part of managing pediatric AML, providing key information on the effectiveness of treatment and the risk of relapse.

OBJECTIVE:

Identify the main molecular biomarkers associated with the early diagnosis and monitoring of MRD in pediatric AML.

MATERIALS AND METHODS:

This study is a rapid literature review, focusing on articles published between 2022 and 2025 on molecular biomarkers in pediatric AML. The articles identified were compiled and analyzed in a 'review matrix', with the results extracted for analysis. This time frame was chosen to capture the latest recommendations from the World Health Organization (WHO), the International Consensus Classification (ICC), and the European Leukemia Net (ELN) regarding the diagnosis and monitoring of AML.

RESULTS:

Pediatric AML is characterized by a diversity of molecular alterations that directly influence the diagnosis, risk stratification, and disease monitoring. Genes such as ASXL1, BCOR, CEBPA, DDX41, EZH2, FLT3-ITD, FLT3-TKD, IDH1, IDH2, NPM1, RUNX1, SF3B1, SRSF2, STAG2, TP53, U2AF,1 and ZRSR2 (Genes necessary for diagnosis and risk stratification) are essential for initial assessment and for defining prognostic risk. In addition, the presence of structural variants such as BCR::ABL1, CBFB::MYH11, DEK::NUP214, MECOM::R, KMT2A::R, NUP98::R, RUNX1::RUNX1T1, and PML::RARA also plays a crucial role in the molecular classification of pediatric AML. Recent studies highlight the importance of mutations in genes such as NPM1, FLT3-ITD, RUNX1, ASXL1, SF3B1, SRSF2, STAG2, U2AF1, and ZRSR2 (Additional genes recommended for diagnostic

testing and disease monitoring) in the risk stratification of pediatric AML. Mutations in NPM1 and FLT3-ITD are associated with an intermediate risk, while mutations in RUNX1, ASXL1, SF3B1, SRSF2, STAG2, U2AF1, and ZRSR2 are indicative of an adverse risk. This information is essential for personalizing treatment and monitoring MRD to improve clinical outcomes in pediatric patients with AML.

CONCLUSION:

Molecular biomarkers are promising tools for risk stratification and personalized therapy in pediatric AML. Ongoing prospective studies aim to validate their routine clinical application.

KEYWORDS:

Acute Myeloid Leukemia; Minimal Residual Disease and Pediatric Patients.

IDENTIFICATION OF THE NOVEL HLA-A*02:1187N ALLELE USING NEXT-GENERATION SEQUENCING IN BRAZILIANS BONE MARROW DONORS

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INTRODUCTION:

Next-Generation Sequencing (NGS) plays a crucial role in identifying HLA null alleles, which are non-functional alleles that do not produce a functional protein. These alleles are marked with an "N" and can be crucial for accurate HLA typing in transplantation. NGS's high-resolution capabilities allow for the detection of novel null alleles that might be missed by traditional methods, leading to better donor-recipient[1]. Next-generation sequencing (NGS) has allowed the identification of a new HLA-A null allele, as HLA-A*02:1187N.

METHODS:

Genomic DNA extraction was obtained from peripheral blood using the Extracta Station 9600 instrument and the MHLA-PU16S kit (LOCCUS, BRAZIL). DNA concentration was quantified using the Qubit dsDNA broad range (BR) assay kit (Invitrogen, Waltham, MA). The AllType NGS kit, 11 loci (One Lambda, Canoga Park, CA) was used to prepare the library of HLA-A,-B,-C,-DRB1, -DRB345,-DQB1,-DQA1,-DPB1, and -DPA1 loci. This novel HLA-A null allele was identified in a Brazilian marrow donor using NGS. The MiniSeq genomic sequencer (Illumina, San Diego, CA) was used to sequence the library preparation. The novel HLA-A*02:1187N allele was analyzed using the TypeStream Visual NGS 4.6 analysis software (One Lambda, Canoga Park, CA).

RESULTS:

This allele HLA-A*02:1187N was characterized by a mutation that disrupts protein expression, often resulting in a premature stop codon. It differs from the closely related HLA-A*02:01:01:01 allele by a deletion of the Guanine base in exon 2 at position 280 of the gDNA. This deletion creates a premature stop codon, resulting in a truncated protein and a nonfunctional HLA-A molecule.

CONCLUSION:

NGS provides a powerful tool for HLA typing, allowing for the identification of novel alleles, including null alleles. Its high-resolution capabilities enable the detection of subtle sequence variations, including those that lead to null allele formation. Null alleles can have significant implications in various fields, including transplantation. Understanding the prevalence and characteristics of null alleles is crucial for optimizing these fields. The nucleotide sequence of this novel allele has been submitted to the GenBank database under accession number PP994769. The name A*02:1187N was officially assigned by the WHO Nomenclature Committee for HLA System Factors in August 2024[2].

KEYWORDS: HLA, null allele, NGS

MULTIPROFESSIONAL ACTION TO GUARANTEE THE RIGHT TO EDUCATION IN HIGHLY COMPLEX TREATMENTS

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INTRODUCTION:

TheprojectMultidisciplinarywork,sociodemographic and emotional profile and school performance: qualitative and quantitative assessment in an Onco-hematology service highlights the importance of multidisciplinary work in highly complex treatments and presents results related to the educational area in interface with health (Hospital Schooling Program SME/CURITIBA and Hospital Schooling Network Support Service SAREH/SEED/PR).

OBJECTIVE:

To demonstrate the importance of multidisciplinary work in guaranteeing the right to education for children/young people with hemato-oncological diagnoses associated with autism (7), intellectual disability (12) and significant learning delay (52) and adrenoleukodystrophy (14).

CASE SERIES:

Onco-hematology and Hematopoietic Stem Cell Transplantation (HSCT) patients. Sampling: 85 student-patients (March/2021 to May/2025). Median age: 7.3 years (Preschool; Elementary School I), 15.5 years (Elementary School II; High School). Main diagnoses: oncological diseases and rare diseases.

METHOD:

Descriptive-explanatory with a qualitative-quantitative approach, with a participant-type intervention research design.

RESULTS:

They reveal the importance of educational programs that guarantee the right to education in pediatric hospitals, strengthening school ties and, notably, ensuring the learning of children/young people with specific educational needs, recognizing that treatment affects schooling due to constant absences and consequences (direct and indirect) of this. The multidisciplinary work favored the construction of pedagogical plans where each child/young person was perceived in their singularity, contemplating specific needs by intertwining curricular content with the context of life. Issues such as oral and hand hygiene, nutrition, capacity measurements (medication dosage), name/function of the main medications, were class topics, reinforcing health care by intertwining hospital experience and education. producing meaningful and motivating learning.

CHALLENGES:

personalized care in response to demand; reintegration into school, maintaining personalized teaching plans and meeting treatment requirements (sun exposure, nutrition, attitudinal support), a situation aggravated by low parental education and precarious support networks. Conclusions: There is a need to increase the number of education programs in Brazilian pediatric hospitals, strengthening partnerships with health teams and education systems, with the aim of building a structured support network that meets the specific needs of those undergoing highly complex health treatment.

KEYWORDS:

Multidisciplinary Work – Research-Intervention – Educational Inclusion

OVERVIEW OF BONE MARROW TRANSPLANTATION IN THE BRAZILIAN PUBLIC HEALTH SYSTEM

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INTRODUCTION:

Brazil's public healthcare system provides medical care to all citizens, including bone marrow transplantation. This study analyzes publicly available data from 2023, in a post-pandemic setting, to assess the current state of bone marrow transplantation in the country.

OBJECTIVES:

To identify weaknesses in the system and propose solutions to ensure access to treatment for all individuals, regardless of geographic location or socioeconomic status.

METHODS:

In June 2024, data were extracted from the Hospital Information System available on the Brazilian Ministry of Health's DATASUS platform. We reviewed records of hospitalizations for bone marrow transplantation performed in public healthcare facilities in 2023. Data were tabulated using the Ministry of Health's Tabwin software. We analyzed the total number of transplants nationally and regionally, categorized by procedure type and distribution. We reviewed the number of transplants performed at each center and calculated states' transplantation indexes, defined as the number of procedures per ten million inhabitants by the World Health Organization. Descriptive and comparative analyses were conducted using Microsoft Excel, with results presented through statistical summaries and graphical representations.

RESULTS:

The public system accounted for 2,990 transplants in 2023, 1851 autologous and 1,108 allogeneic. These represent 70% of transplants in the country, according to the total number of bone marrow transplants published by the Brazilian Society of Organ Transplant for 2023. The national transplantation index for the public system was 208, with rates of 130 for autologous and 78 for allogeneic transplants. Only 15 from the 27 federative units of the country performed transplants through the public system, with indexes varying from 21- 100 (image 1). None of the states reached the 500 index, the ideal expected in developed countries, as illustrated in the figure below.

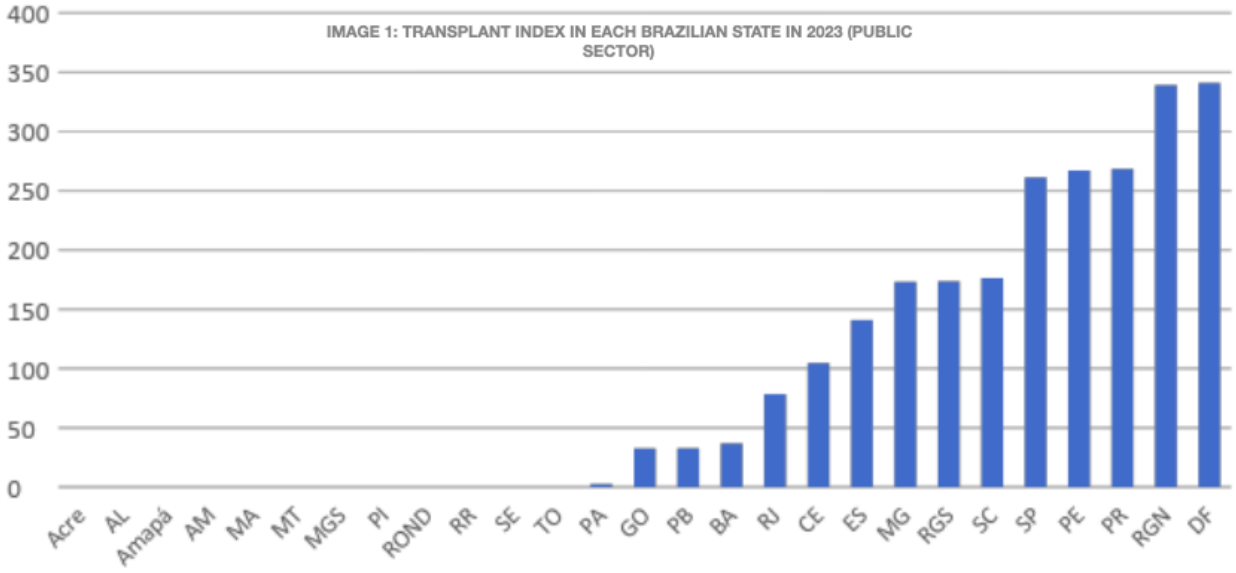
Image number 2 illustrates the expected number of transplants based on the 500 index goal for each Brazilian state, compared with the real absolute number of transplants performed in 2023.

CONCLUSION:

Although the number of bone marrow transplants has increased over the past decade, major disparities in access remain. In 2023, twelve states did not perform any transplants. Addressing this issue requires a stronger regionalization approach within the national public healthcare system. The figure below shows the number of transplants carried out in each Brazilian state during 2023.

Even in states with higher transplant activity, such as São Paulo (261) and Paraná (268), the transplant index is still lower than global benchmarks. The states with the largest difference between actual and expected numbers were Rio de Janeiro, Bahia, Minas Gerais, and São Paulo. All of these have medical teams and hospitals capable of handling more procedures. With appropriate investment and strategy, these states could deliver the required care.

KEYWORDS: Bone Marrow, Transplantation, Index



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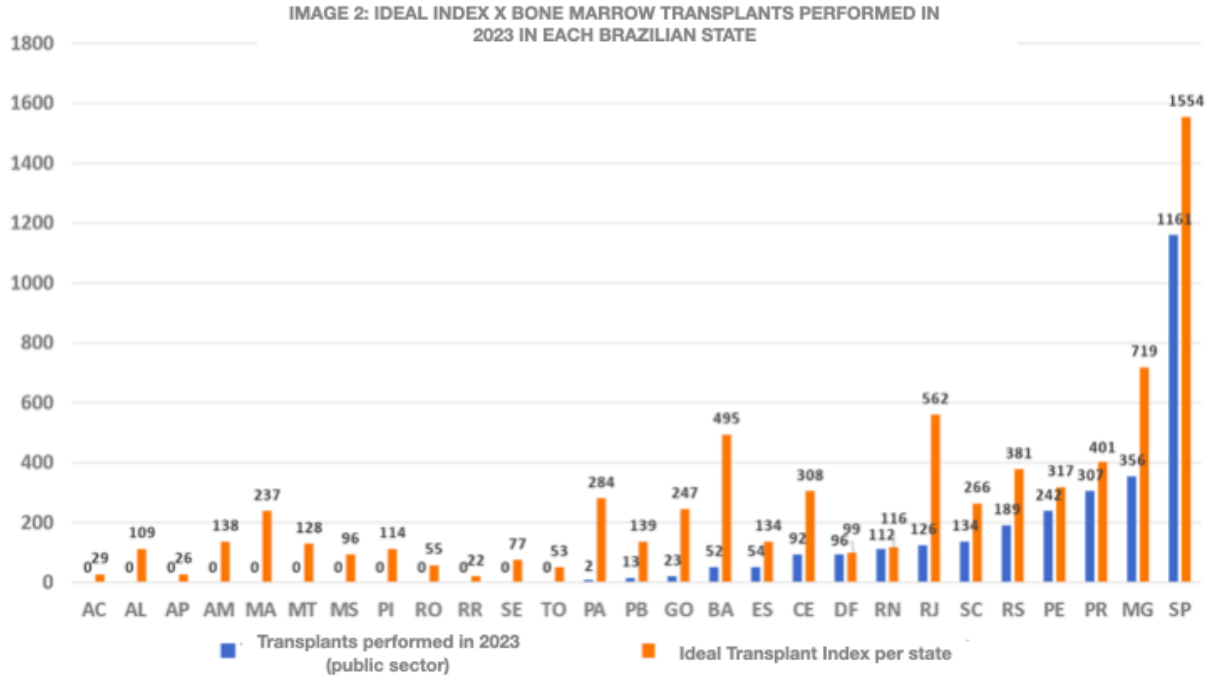

IMAGE 3: NUMBER OF BONE MAROW TRANSPLANT PER STATE IN BRAZIL

STRUCTURING A SURVIVORSHIP PROGRAM FOR PATIENTS UNDERGOING HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Cancer survivorship programs aim to promote long-term quality of life, prevent and manage late complications, facilitate social reintegration, and provide emotional support. This follow-up care contributes to a reduction in severe complications, improved treatment adherence, and overall well-being. Patients undergoing hematopoietic stem cell transplantation (HSCT) are particularly susceptible to long-term adverse effects such as graft-versus-host disease (GVHD), recurrent infections, organ dysfunction, secondary malignancies, and psychosocial challenges. Long-term follow-up enables proactive surveillance and early intervention, which are essential to mitigate these risks.

OBJECTIVE:

To present the main strategies adopted in the structuring of a survivorship program for patients undergoing HSCT.Methods: The program is being structured through a participatory and multidisciplinary process involving professionals from hematology, nursing, psychology, nutrition, physiotherapy, and integrative medicine, with institutional leadership support. Development is occurring in four main phases, of which the first three have been completed and the fourth is currently in progress: (1) Situational analysis: literature review and institutional data analysis focused on the needs of post-HSCT patients. (2) Needs assessment: collection of case reports and clinical experiences from the care teams to identify priority demands of survivors. (3) Design of the care model: definition of program objectives, inclusion criteria, care pathways, consultation frequency, follow-up indicators, assessment tools, and the creation of an institutional support policy. (4) Validation and pilot implementation: review and approval of the model by clinical and administrative leadership, followed by the launch of the pilot phase. All processes are based on national and international guidelines, adapted to the institution's context and resources.

RESULTS:

The structuring process has resulted in the development of a dedicated care protocol for post-HSCT follow-up, including a consultation schedule, clinical monitoring criteria, standardized quality-of-life assessment tools, screening for late effects, and multiprofessional care guidelines. Interdepartmental referral flows have been defined, as well as the care team's composition and a detailed institutional policy. The pilot phase is currently underway and is expected to include all late-phase post-HSCT patients in the coming months.

CONCLUSION:

The structuring of a survivorship program for post-HSCT patients, based on evidence and tailored to institutional realities, represents a significant advancement in the longitudinal care of this population, with the potential to reduce complications and enhance long-term quality of life.

KEYWORDS:

Hematopoietic Stem Cell Transplantation, Cancer Survivors, Long-Term Care

STRUCTURING INDICATORS OF COLONIZATION BY MULTIDRUG-RESISTANT BACTERIA IN ONCO-HEMATOLOGY AND HEMATOPOIETIC STEM CELL TRANSPLANTATION UNITS

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INTRODUCTION:

Onco-hematologic patients and those undergoing hematopoietic stem cell transplantation (HSCT) represent a high-risk group for infections, particularly those caused by multidrug-resistant (MDR) bacteria. It is known that, among immunosuppressed patients—especially those with neutropenia—colonization by MDR organisms can increase the risk of infection by up to 38 times compared to non-colonized individuals. Systematic screening for colonization in this population, along with continuous monitoring of new cases, is essential to support infection prevention and control strategies, as well as to guide the early selection of appropriate empirical antimicrobial therapy.

OBJECTIVES:

To structure and implement indicators for colonization by MDR bacteria in onco-hematology and HSCT units, including the positivity rate of admission and weekly surveillance cultures.

METHODS:

This is a descriptive study on the development and implementation of colonization indicators for MDR bacteria in a high-complexity private tertiary hospital in São Paulo, Brazil. Until 2022, systematic screening for MDR colonization was performed in these units; however, surveillance did not distinguish between admission and hospital-acquired cases, and there was no consolidated evaluation of all new hospital-acquired colonization events. Data were obtained from the electronic database of the Hospital Infection Control Service (HICS) and organized in a dedicated spreadsheet. Two indicators were created: (1) positivity rate of admission surveillance cultures and (2) positivity rate of weekly cross-sectional surveillance cultures (positive swabs/total swabs × 100).

RESULTS:

A dedicated database was created for surveillance cultures collected in the onco-hematology and HSCT units. The database was configured to extract numerators and denominators by collection unit and time between admission and sample collection. Cultures were stratified into admission and cross-sectional categories. Positive cases were categorized by type of isolated agent (e.g., carbapenem-resistant Klebsiella spp., vancomycin-resistant Enterococcus, carbapenem-resistant Acinetobacter spp., carbapenem-resistant Pseudomonas spp., and other carbapenem-resistant Enterobacteriaceae) and by type of collection (admission or cross-sectional).

CONCLUSION:

The structuring and implementation of colonization indicators for MDR bacteria in onco-hematology and HSCT units enabled the identification of positivity rates at different stages of care, providing greater clarity on the microbiological profile of these populations. Knowledge of prior colonization allows for targeted preventive actions, enhances epidemiological surveillance, and supports the selection of more appropriate empirical antimicrobial therapy. Next steps include monitoring temporal trends of these indicators, correlating them with infection rates, and evaluating the clinical impact of implemented interventions.

KEYWORDS: Bacterial colonization, Multidrug resistance, Onco-hematology

THE IMPACT OF BONE MARROW TRANSPLANTS PERFORMED THROUGH A PUBLIC-PRIVATE PARTNERSHIP PROJECT ON EQUITY IN ACCESS TO THE TREATMENT OF SEVERE DISEASES IN BRAZIL: REAL-WORLD EVIDENCE

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INTRODUCTION:

Hematopoietic Stem Cell Transplantation (HSCT) has become established as a highly sophisticated scientific and technical treatment for several serious diseases. In this context, considering that Brazil is a country with significant disparities in the distribution of high-complexity healthcare services, it is evident that the majority of HSCT centers are concentrated in the Southeast-South axis. This concentration exacerbates inequities in access to bone marrow transplantation (BMT), particularly for patients from other regions of the country.

OBJECTIVE:

To analyze the impact of bone marrow transplants performed through a public-private partnership project on the equity of access to treatment for serious diseases in Brazil.

CASE SERIES:

The study included all patients who underwent either autologous or allogeneic bone marrow transplantation from 2022 to 2025.

METHOD:

This is a retrospective descriptive study based on Real World Evidence (RWE), considering data on origin, diagnoses, age, and types of HSCT performed on patients from the Brazilian Unified Health System (SUS) through a public-private partnership project of the Program to Support the Institutional development of the SUS (PROADI-SUS) between June 2022 and March 2025. Real-world data (RWD), including demographic and clinical information, were collected from a REDCap platform database after signing an informed consent form for participation in research.

RESULTS:

During the study period, 13 autologous and 65 allogeneic BMTs were performed through the project, totaling 78 procedures in patients aged between 2 and 71 years (median age: 35.5 years). Notably, patients came from various states across the country, ranging from those without a registered transplant center to better-structured states that nonetheless face a shortage of transplant slots for specific populations, such as pediatric patients (Figure 1). The project also treated a wide range of diagnoses, including both hematological and solid organ diseases, as well as oncological and non-oncological conditions (Figure 2).

CONCLUSIONS:

It is concluded that the availability of strategic beds for HSCT through the public-private partnership project has a significant impact on increasing access to this type of procedure in regions with greater technological and socioeconomic development disparities. In this context, it is essential to strengthen and promote similar projects in order to support SUS and reduce transplant waiting times. Additionally, it is important to invest in the training of Hematology services across the country to further expand access to BMT.

KEYWORDS: Bone Marrow Transplantation; Equity in Access to Health Services; Unified Health System.

FIGURE 1.

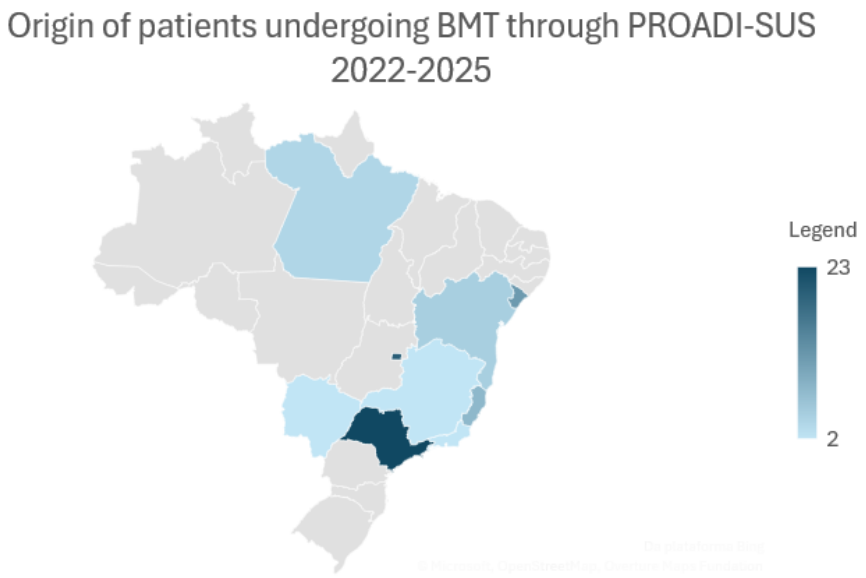
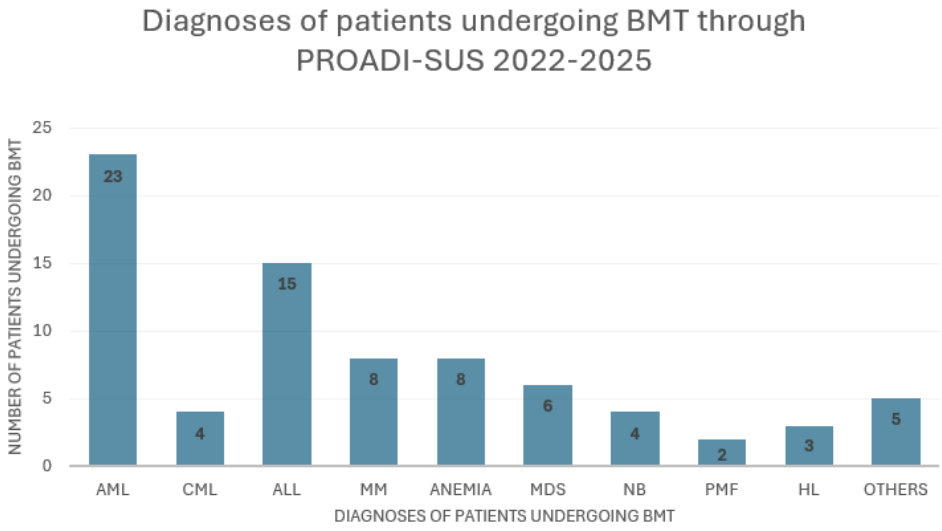


FIGURE 2.



AML = Acute Myeloid Leukemia; CML = Chronic Myeloid Leukemia; ALL = Acute Lymphoblastic Leukemia; MM = Multiple Myeloma; MDS = Myelodysplastic Syndrome; NB = Neuroblastoma; PMF = Primary Myelofibrosis; HL = Hodgkin's Lymphoma

VALIDATION OF HEMATOPOIETIC STEM CELL (CD34+) QUANTIFICATION USING THE FACSVERSE FLOW CYTOMETER

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INTRODUCTION:

Enumeration of circulating CD34+ cells by flow cytometry is critical for determining the optimal timing for hematopoietic stem cell (HSC) collection in autologous transplantation, as well as for evaluating the required CD34+ cell dose in the apheresis product for infusion. Therefore, validating the performance of the flow cytometer is essential to ensure the accuracy and reliability of this analysis.

OBJECTIVE:

To validate the quantification of CD45+/CD34+ cells by flow cytometry using the dual-platform ISHAGE method on the FACSVerse cytometer.

METHODS:

Twenty-four samples—comprising 12 mobilized peripheral blood specimens and 12 leukapheresis products—were analyzed using both a reference flow cytometer (BD FACSCalibur) and the FACSVerse cytometer, which was undergoing validation, to determine the percentage of CD34+ cells. The protocol followed the guidelines established by the International Society for Hematotherapy and Graft Engineering (ISHAGE). Data obtained from both instruments were subjected to statistical analysis and compared to assess agreement in CD34+ cell enumeration. The study was submitted to and approved by the institution's CAPPq and Research Ethics Committee (CEP), under protocol number 6.939.773.

RESULTS:

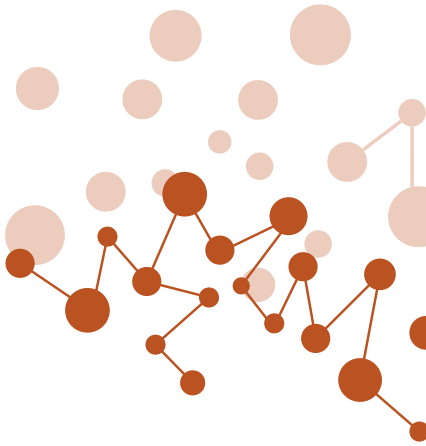
The mean percentage of CD34+ cells identified was 0.22% (range: 0.01–0.86) with the FACSVerse cytometer and 0.23% (range: 0–0.72) with the reference cytometer. Spearman's test revealed a strong and statistically significant correlation in CD34+ cell quantification between the two instruments ($r = 0.99$; $p < 0.0001$). Bland-Altman analysis demonstrated good agreement between the cytometers, with no significant bias observed for either dataset ($p > 0.05$).

CONCLUSION:

The results suggest that the FACSVerse cytometer is comparable to the reference instrument for CD34+ cell quantification. However, an increased number of tests and analyses are necessary to ensure the release of results with greater safety and reliability. Although the FACSVerse cytometer is not currently registered with ANVISA, the data obtained from this instrument may be used as preliminary results prior to official release by the supporting laboratory, thereby aiding in the organization and planning of multidisciplinary teams involved in patient care.

KEYWORDS: Cellular Processing Center; Flow Cytometry; Hematopoietic Stem Cells.

QUALITY AND DATA MANAGER



ADVANCED CELL THERAPY AND CONVENTIONAL MANAGEMENT: DASHBOARD IMPLEMENTATION BY THE ANALYTICS TEAM

Cinthya Corrêa da Silva[1], Bruno Rizzato Coelho Pina[1], Edson Dias[1] , Katherine Warwick Parker de Sa Leitao[1] , Gabriela Renata Raci[1] ,Graziely Dias de Oliveira [1], Daniel Almeida[1], Gustavo Rezende[1], Henrique Maresca Nascimento[1], Giovanna da Silva Avelino[1], Daniela Vivas dos Santos[1], Gabriela Rocco de Sa[1], Camila Viale Nogueira[1], Carolina da Silva Costa[1] , Andrea Tiemi Kondo[1], Vanessa Damazio Teich[1] , Juliana Todaro[1] , Lucila Nassif Kerbauy[1], Jose Mauro Kutner[1] , Nelson Hamerschlak[1]

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INTRODUCTION:

Integrating an analytics team within the Hematopoietic Cell Transplantation (HCT) and Advanced Cell Therapy (ACT) Unit is essential to optimize clinical operations and support data-driven decisions. Dashboards for conventional HCT and ACT enable systematic monitoring of procedurals, indications, and complications. Objective: Describe the structured development and implementation of dashboards for conventional HCT and ACT during 2024, highlighting surveillance, resources allocation and challenges.

METHODS:

Data was consolidated from the institutional database, data lake and spreadsheets into a shared drive. An automated Extract, Transform, Load workflow was developed using Python scripts for a standardized process with daily updates. An analytics specialist created SQL queries to extract relevant fields, integrated via direct queries into Power BI for real-time visualization. Dashboards were deployed on the institutional server with scheduled updates and controlled access provided to employees for continuous monitoring, (Figure 1).

RESULTS:

The analytics team successfully developed and made available 123 operational dashboards for the institution (51 oncology/hematology and 72 robotic, surgery and medical intelligence). With 0.3 thousand users, 0.2 thousand total dashboards accessed, and 132 thousand total accesses. Among the dashboards developed were some for the HCT and ACT's visualizations that hadn't existed previously, but through access management 17 individuals accessed the HCT dashboard, with 504 total consults, while 22 accessed the CAR-T dashboard, with 270 consults. When evaluating the profiles who consumed data from the HCT and CAR-T dashboards, 82% to 88% (15 to 18 users) belonged to the multidisciplinary team. Operational and clinical managers from the oncology and hematology team used the data strategically to assess the number of procedures, comparing them with previous months and years. Plan operational and clinical staffing and correlate them with dashboards such as financial panels. The data also provided support for research development, enabling quick and real-time queries for administrative issues, not being specifically for clinical challenges, which includes volumetrics, commercial aspects, or research-related information such as cell types. Immediate access was available to team members, aiding in staffing dimensioning, correlating with clinical indicators and other unit performance metrics, (Table 1).

CONCLUSIONS:

Visualization of institutional indicators enabled systematic monitoring of clinical outcomes, supporting continuous improvements. The dashboards streamlined workflows and enhanced data accessibility, integrating into the operational management, procedural oversight, and team coordination. Additionally, the data provision process underscored the importance of shared responsibility for data accuracy, while dashboard deployment improved institutional information's transparency.

KEYWORDS: Dashboards, cell therapy, data analytics.

ANALYSIS OF THE PROFILE OF PATIENTS UNDERGOING BONE MARROW TRANSPLANTATION IN A CAPITAL CITY OF THE AMAZON REGION

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INTRODUCTION:

Bone marrow transplantation (BMT) is a therapeutic strategy associated with remission and improved progression-free survival in various hematologic malignancies and non-malignant disorders. The Amazon region, despite being the most biodiverse area in the world, experiences major socioeconomic constraints, including limited access to healthcare services, particularly in specialized areas such as bone marrow transplantation.

OBJECTIVE:

To analyze the demographic and clinical profile of patients undergoing BMT at a hospital located in a capital city in the Amazon region.

MATERIALS AND METHODS:

This is a retrospective, cross-sectional, analytical study based on secondary data, with a quantitative and descriptive approach. The study population consisted of patients who underwent autologous or allogeneic BMT between May 2022 and April 2025 at a reference center in the Amazon region.

RESULTS:

A total of 52 BMTs were performed: 49 autologous and 2 allogeneic. The mean age of patients was 55.3 years-old, with a predominance of females (62.7%). Among autologous transplants, the main indications were Multiple Myeloma (73.4%), Non-Hodgkin lymphoma (16.3%), and Hodgkin lymphoma (8.1%). Of the allogeneic transplants, 50% were indicated for acute lymphoblastic leukemia and 50% for acute myeloid leukemia, all with related donors.

There was an upward trend in transplant activity: 7 autologous transplants in 2022, 16 in 2023, 18 autologous and 2 allogeneic in 2024, and 8 autologous procedures in the first four months of 2025, with further allogeneic transplants scheduled.

Fresh cell infusions accounted for 45% of cases, while 55% required cryopreservation. Neutrophil engraftment occurred on average by day 10. The overall mortality rate was 1.9%, due to infectious complications during the aplastic phase.

CONCLUSION:

Despite significant healthcare challenges in the Amazon region, the bone marrow transplantation program shows continuous growth in procedural volume and scope of pathologies treated. Future perspectives include the incorporation of non-malignant conditions and improvements in patient access to transplantation services.

AVERAGE TIME REQUIRED FOR DATA ENTRY OF BRAZILIAN PATIENTS INTO THE CENTER FOR INTERNATIONAL BLOOD AND MARROW TRANSPLANT RESEARCH (CIBMTR®) REGISTRY

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INTRODUCTION:

Including patients in the Center for International Blood and Marrow Transplant Research (CIBMTR®) platform has become a routine practice in most Brazilian hematopoietic stem cell transplant centers. To carry out this inclusion, a specific amount of time is required during a typically long workday, often divided among various tasks, one of which is data management, including entering patient information into the CIBMTR® registry. Therefore, knowing how long this task takes can help improve productivity, contribute to meeting goals, and ensure deadlines are met.

OBJECTIVE:

To determine the average time required for entering patient data into the CIBMTR®.

METHODS:

This is a time-motion (chronoanalysis) study. A stopwatch was used to measure the time taken to complete each data entry form. The shortest and longest times for each form type were excluded. The following forms were evaluated: assign CRID, consent tool, 2814, 2400, and 2402. The researcher responsible for completing the forms has over 10 years of experience in this activity and an intermediate level of English. All medical records were electronic. Variations in internet connectivity were not considered.

RESULTS:

A total of 60 forms were evaluated, 12 of each type. The average time for completing the assign CRID form was 1 minute and 38 seconds; for the consent tool, 27 seconds; for form 2814, 35 seconds; for form 2400, 25 minutes and 28 seconds; and for form 2402, 12 minutes and 37 seconds. Considering all these initial forms, the total time was 40 minutes and 44 seconds. Factors such as diagnosis and conditioning regimen may influence the time required to complete the forms.

CONCLUSIONS:

Knowing the average time needed for entering and updating patient data in the CIBMTR® platform allows data managers to optimize the time allocated for this activity, making it more productive and contributing to the maintenance of updated data and the generation of productivity indicators for each center. Familiarity with the forms and having organized data beforehand can facilitate the data entry process, as can training related to this task.

KEYWORDS: Data management, Hematopoietic stem cell transplantation, CIBMTR registry.

CHALLENGES AND RESULTS OF LEADING THE DATA MANAGERS GROUP OF THE BRAZILIAN SOCIETY OF BONE MARROW TRANSPLANTATION AND CELLULAR THERAPY

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12 Centro de Transplante de Medula Óssea, Instituto Nacional de Câncer, Rio de Janeiro, RJ, Brazil
13 Hospital das Clínicas da Faculdade de Medicina da Universidade de São Paulo, São Paulo - SP – Brasil
14 Real e Benemérita Sociedade de Beneficência Portuguesa de São Paulo, São Paulo - Brasil
15 Associação Hospitalar Moinhos de Ventos, Porto Alegre, RS
16 Fred Hutchinson Cancer Center and the University of Washington, Seattle, Washington, USA
17 Universidade de Campinas, Campinas, SP-Brasil
18 Center For International Blood And Marrow Transplant Research (CIBMTR) and Medical College of Wisconsin, Milwaukee, WI, - Estados Unidos da América
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INTRODUCTION:

Participation in a professional society is voluntary and coordinating such a group in the field of Cellular Therapy (CT) involves technical, ethical, and organizational challenges. Data managers are vital in handling clinical and laboratory data for therapies like Hematopoietic Cell Transplants (HCT) and CAR-T cell therapies (CAR-CT). With the creation of the Brazilian Registry of Hematopoietic Stem Cell Transplantation and Cellular Therapy (RBTCH-TC) in 2019 by the SBTMO, the Data Managers Working Group (GTGD-SBTMO) was officialized to lead and define national guidelines and protocols.

OBJECTIVE:

To describe the organization, outcomes, and identify the main challenges of coordinating the GTGD-SBTMO, emphasizing its impact on quality and safety of cellular therapies in Brazil and allowing other countries to follow this similar successful model.

METHODS:

Identification of the GTGD-SBTMO activities over time based on meeting records, experiences, training projects, and collaborations.

RESULTS:

Officialized in 2019, the GTGD-SBTMO quickly defined its mission, vision, values, and logo. To meet growing demands, it created subcommittees for administrative, scientific, coordination, data analysis, educational, and international areas (Figures 1,2). The group formalized its structure through Internal Regulations, improving transparency and efficiency in managing RBTCTH-TC data and setting up a model for all SBTMO working groups. Trello was adopted for task management and AI supports monthly meeting records. Since 2016, the group has held 10 in-person meetings at the congress of the SBTMO and 59 educational online events with high satisfaction. In 2017, a multicenter study began with 10 centers, currently,92 centers have been approved by the Brazilian National Research Ethics Commission to submit data to the Center for International Blood and Marrow Transplant Research. While not all have local Ethics Committee approval, they are authorized to operate in Brazil. Of these, 45 centers are active and compliant with regulatory. The group publishes annual "Summary Slides" for HCT and CT, has produced 5 HCT and 2 CAR-CT papers and supports the SBTMO Study Group (GEDECO) with Brazilian registry data. By 2024, it handled 71 database queries, mostly from physicians (82%), with a 92% resolution rate, and 63% aimed at scientific meeting presentations, reflecting its research impact. Initiatives like the "Knowledge Trail for Data Managers" and a Data Management Guide (translated into Spanish) further support data managers (Figure 4). Ongoing challenges include limited resources, coordinator training, and adapting to complex protocols and regional needs.

CONCLUSION:

Managing volunteer data managers in CT requires a multifaceted approach, focusing on continuous training, infrastructure investment, and engagement strategies. These efforts are essential to consolidate the progress and ensure timely, accurate contributions to the advancement of CT in Brazil.

KEYWORDS: cellular therapy; data management; data managers group

FIGURE 1. ORGANIZATIONAL STRUCTURE OF THE DATA MANAGER WORKING GROUP (GTGD)

FIGURE 2. FUNCTIONS OF EACH COMMITTEE

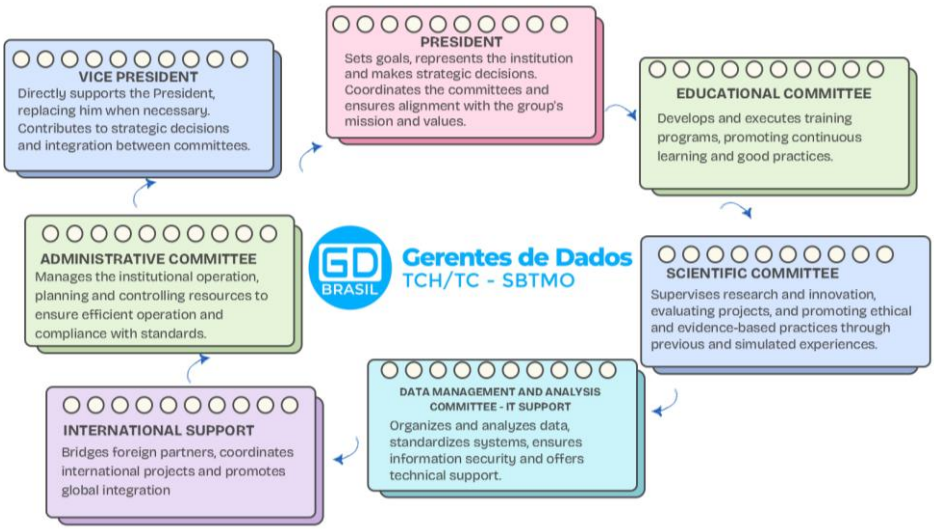

FIGURE 3. TASK ORGANIZATION AND DEADLINES

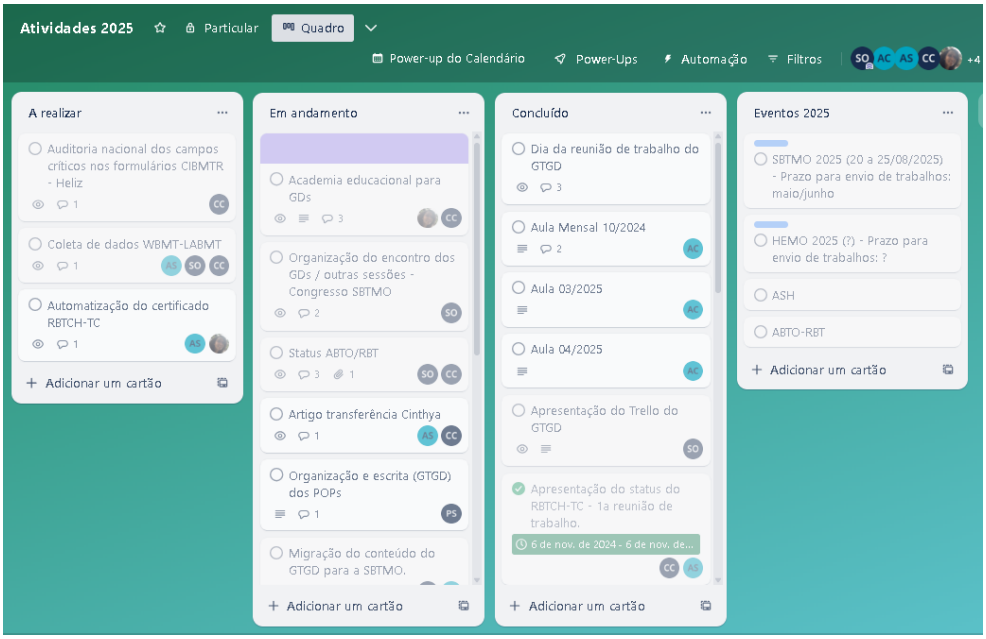

FIGURE 4. SUMMARY OF MAIN ACTIONS

CLINICAL VALIDATION OF A POINT-OF-CARE DEVICE VERSUS STANDARD MONITORING OF VITAL SIGNS IN PATIENTS UNDERGOING HEMATOPOIETIC CELL TRANSPLANTATION: A PROSPECTIVE, SINGLE-CENTER PILOT STUDY

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INTRODUCTION:

Changes in vital signs often precede clinical deterioration, enabling timely intervention. This is particularly critical in hematopoietic cell transplantation (HCT), where early and appropriate action can improve patient outcomes. Commercially available wearable and point-of-care sensors are increasingly being investigated as tools to enhance patient safety. Objective: To clinically validate point-of-care monitoring in patients undergoing autologous and allogeneic HCT.

METHODS:

This prospective, single-center pilot study enrolled 20 inpatient HCT recipients (including both autologous and allogeneic HCT), aged 18 years or older, with reliable reading capacity. The BioMonitor device (BioZ4Life) intermittently recorded heart rate (via ECG), blood pressure (mean arterial pressure, MAP), pulse rate (PR), respiratory rate (RR), oxygen saturation (SpO_2), and temperature transmitting data wirelessly to a smartphone. Measurements were taken two to three times daily over 48–72 h per week from pre-transplant until discharge or day 30 post-HCT, whichever occurred first. Vital signs were sequentially measured by an investigator using both the BioMonitor and validated standard equipment (gold standard). The primary outcome was the agreement between the two methods, assessed using the Bland and Altman method to calculate mean bias with 95% limits of agreement (LoA). Acceptable LoA thresholds were defined as: ±0.5°C for temperature, ±3% for SpO_2, ±5 bpm for HR/PR, ±3 rpm for RR, and ±10 mmHg for MAP.

RESULTS:

Of the 20 patients, 4 (20%) underwent allogeneic HCT and 16 (80%) autologous HCT, with a median age of 34 years. Diagnoses included myelodysplasia, acute leukemia, Hodgkin lymphoma, non-Hodgkin lymphoma, and plasma cell disorders. Febrile neutropenia occurred in 94% of patients; sepsis was observed in one allogeneic HCT recipient. One allogeneic HCT recipient died due to graft failure; no deaths or relapses were recorded in the autologous group. A total of 1,244 pairs of vital sign measurements were collected. The mean bias and LoA between the two devices were as follows: temperature -0.37°C (LoA +0.83 to -1.58); PR +0.49 bpm (+46.2 to -45.22); SpO_2 +1.8% (+8.75 to -5.15); MAP -2.16 mmHg (+14.46 to -18.78); RR -0.23 rpm (+4.34 to -4.81); and HR -3.26 bpm (+7.83 to -14.34).

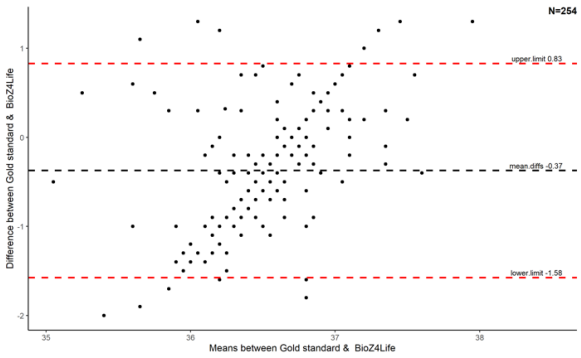
CONCLUSION:

Point-of-care sensors may help detect early clinical deterioration and support remote monitoring in HCT patient. Despite their advantages, the LoA for temperature and PR rate were not reproducible, whereas for SpO_2, HR, RR, and MAP, the LoA were borderline acceptable. The fact that all measurement pairs were taken sequentially rather than simultaneously may have affected reproducibility. The measurements of SpO_2, HR, RR, and MAP by the BioMonitor warrant further investigation in the outpatient HCT setting.

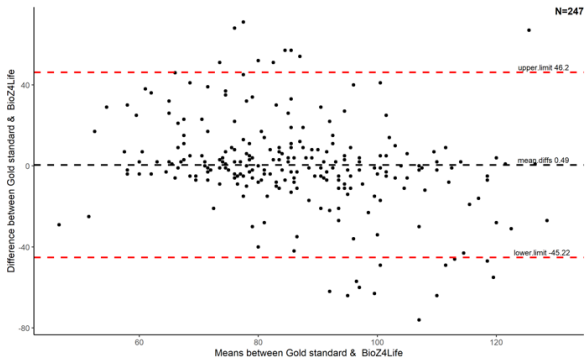
KEYWORDS: Hematopoietic Cell Transplantation (HCT); Point-of-care sensors; Vital signs.

Table 1: Bland-Altman curver for different vital signs with means and 95% limits of agreement

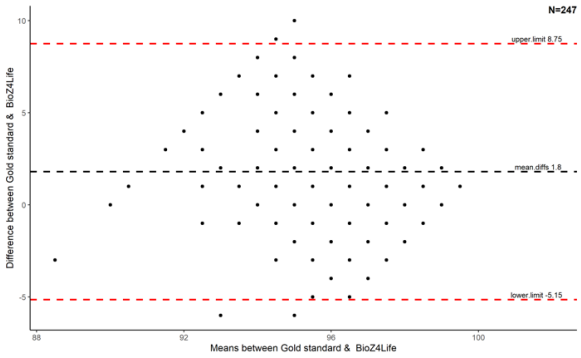
Temperature



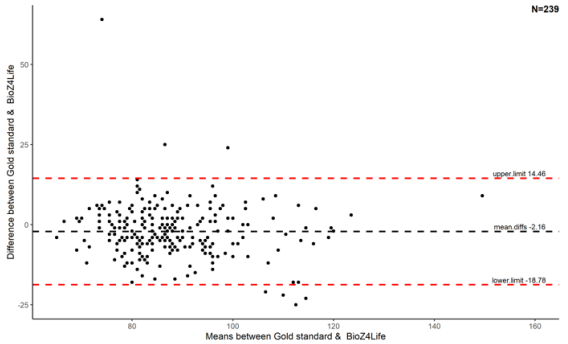
Pulse rate



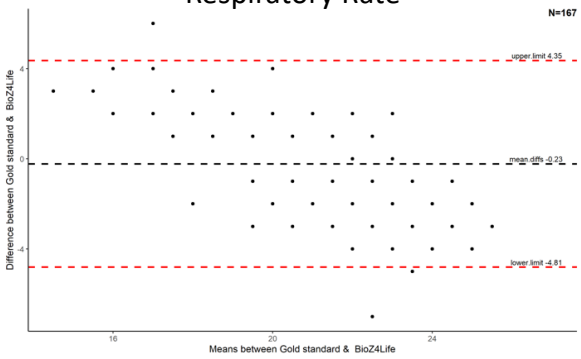
SpO_2



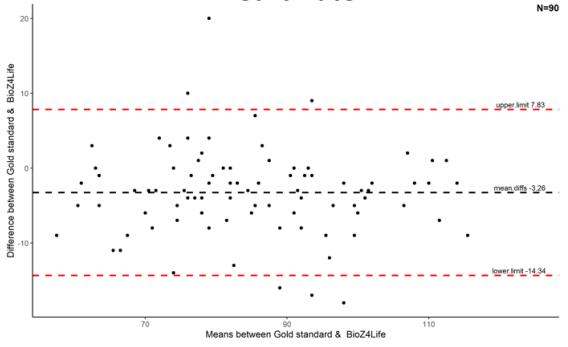
Mean Arterial Pressure



Respiratory Rate



Heart Rate



DATA AUDITING AS A STRATEGY FOR INFORMATION QUALITY IN A HCT CENTER: A CONTINUOUS IMPROVEMENT EXPERIENCE (2024–2025)

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INTRODUCTION:

The accuracy of data submitted to international registries such as the Center for International Blood and Marrow Transplant Research (CIBMTR) is essential for outcome monitoring, institutional performance evaluation, and comparability between centers. In this context, data auditing emerges as a key practice to detect inconsistencies, promote corrections, and support continuous improvement in the processes of Hematopoietic Stem Cell Transplantation (HSCT) care.

Objective: To describe the experience of systematic auditing of clinical data related to HSCT, with emphasis on the qualification of information submitted to the CIBMTR between 2024 and 2025, in a private reference center.

METHOD:

Experience report based on a retrospective analysis of clinical-care data recorded in the CIBMTR. Two distinct audit cycles were evaluated: from August 2023 to August 2024 (n=12 medical records; 776 audited fields) and from January 2024 to April 2025 (n=11 medical records; 537 audited fields). Data were categorized into three groups: compliant, missing, and non-compliant. Based on the analysis, multidisciplinary action plans were developed focusing on improving data collection and entry processes.

DISCUSSION:

In the first cycle, 94.9% compliance was observed in the audited fields, with 4.1% of missing data and 1.0% non-compliance. The main gaps included missing laboratory tests (CMV serology, albumin, ferritin, LDH), genetic tests (FISH, karyotype, NGS), clinical data (Karnofsky score, disease classification and response), and date of diagnosis. In the following cycle, compliance increased to 97.0%, and the rate of errors proportionally decreased, although diagnostic and registration data remained the main weaknesses.

RESULTS:

Implemented actions included: monthly feedback on inconsistencies during team meetings, reinforcement of clinical form submission by the attending physician before hospital admission, and coordination with patient registration and admissions departments to ensure data integrity. The late follow-up outpatient clinic was also consolidated as an important source of post-HSCT data. Improvements between audit cycles demonstrated the positive impact of the action plan: missing data decreased from 4.1% to 2.0%, and non-compliant fields remained at 1.0%, with reduced severity and better traceability of causes.

CONCLUSION:

The experience showed that periodic data auditing, integrated with educational strategies and well-defined workflows, significantly contributes to improving the quality of information reported to the CIBMTR. The engagement of multidisciplinary teams and the institutionalization of feedback practices and continuing education were fundamental to the progress observed. These actions have proven to be sustainable and replicable in other HSCT services seeking excellence in information management.

KEYWORDS: Hematopoietic Stem Cell Transplantation, Data Auditing, Information Quality, CIBMTR, Continuous Improvement, Outcome Monitoring.

DATA MANAGER SUPPORT MANUAL FOR HEMATOPOIETIC CELL TRANSPLANTATION AND CELLULAR THERAPY FOR LATIN AMERICA DEVELOPMENT: AN INNOVATIVE APPROACH TO DATA REPORTING TO THE CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

Flávia Ferreira Costa[1], Cinthya Muniz Corrêa Rocha da Silva[2], Paula Moreira da Silva Sabaini[3]; Anderson João Simione[4], Heliz Regina Alves Das Neves[5], Monique Ammi[6], Rosana Rocha Batista Concilio[7], Afonso Celso Vigorito[8], Eliana Cristina Martins Miranda[8], Leonardo Jun Otuyama[9], Joaquim Gasparini dos Santos[9], Mariza Toledo[10], Adriana Mendes de Quadros Cavilha[5], Jessica Di Chiara Salgado[11], Eudoxiana Melo[12], Simone Ojima Ferreira[13], Antônio Vaz de Macedo[14], Adriana Seber[15], Carmem Maria Sales Bonfim[16], Vaneuza Araújo Moreira Funke[5], Marcelo Pasquini[17], Mary E. Flowers[18], Nelson Hamerschlak[2], Sebastian Galeano[19] , Fernando Barroso Duarte[20]

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INTRODUCTION:

Since 2016, efforts have been underway to consolidate the Brazilian Registry of Hematopoietic Cell Transplantation and Cellular Therapy (HCT-CT/BR) (Figure 1), aiming to enhance the understanding of national transplant practices and outcomes. Created in 2019 through a collaboration between the Brazilian Society of Bone Marrow Transplantation and Cellular Therapy (SBTMO) and the Center for International Blood and Marrow Transplant Research (CIBMTR), the HCT-CT/BR relies on the training and standardization of data managers (DMs) for its sustainability. In this context, a support manual became essential to unify practices in the complex field of HCT and cellular therapy. Objective: To describe the development of a support manual for Brazilian DMs who report data to the CIBMTR and to present the results of a material quality assessment.

METHODOLOGY:

It is a descriptive methodological study carried out in 5 steps: 1. Needs assessment; 2. Content planning; 3. Manual development; 4. Expert validation; 5. Final review. To assess user engagement and satisfaction, a REDCap® survey evaluated access, satisfaction (scale 0–10), clarity, format adequacy, and likelihood of recommendation.

RESULTS:

The Manual was developed between February-August 2024. In phase 1, an analysis of the main difficulties of Brazilian DMs was carried out. At phase 2, the content was structured in 10 topics (Figure 2). Phase 3 counted with experts' voluntary collaboration (physicians, nurses, DMs, analysts, Portuguese-language reviewers, and designers). Right away, the content was subjected to a spelling and instructional design review and validated (phases 4 and 5). The Manual was prepared in digital (Figure 3) and printed formats, 200 copies were printed for launch and sale during the 2024 SBTMO Congress, and the digital version was made available on the SBTMO, CIBMTR, and HEMATOLOG websites. Of the printed copies, 52% (103) were sold at a unit cost of $11.00. Two copies were purchased by institutions in other Latin American countries. Among the 44 HCT/CT centers contributing to the 2024 Brazilian summary, 50% acquired the printed manual. From August 2024 to May 2025, the SBTMO website identified 220 visits to the e-book page, while HEMATOLOG recorded 3,000 views in the same period. The Spanish version of the Manual (Figure 4) was launched in May 2025, the SBTMO website identified 220 visits to the e-book page, while HEMATOLOG recorded 799 views. A total of 39 professionals completed the survey (Figure 5); of these, 29 (74%) purchased the manual (26 in print, 2 digitais, and 1 unspecified). The average satisfaction score was 9.3/10, with a recommendation rating of 9.4/10.

CONCLUSION:

the support manual plays a key role in standardizing data reporting processes and enhancing data quality. The manual helps to comprehend the processes and identify critical stages. Furthermore, its flexibility will allow frequent updates and strengthening the educational process.

FIGURE 1

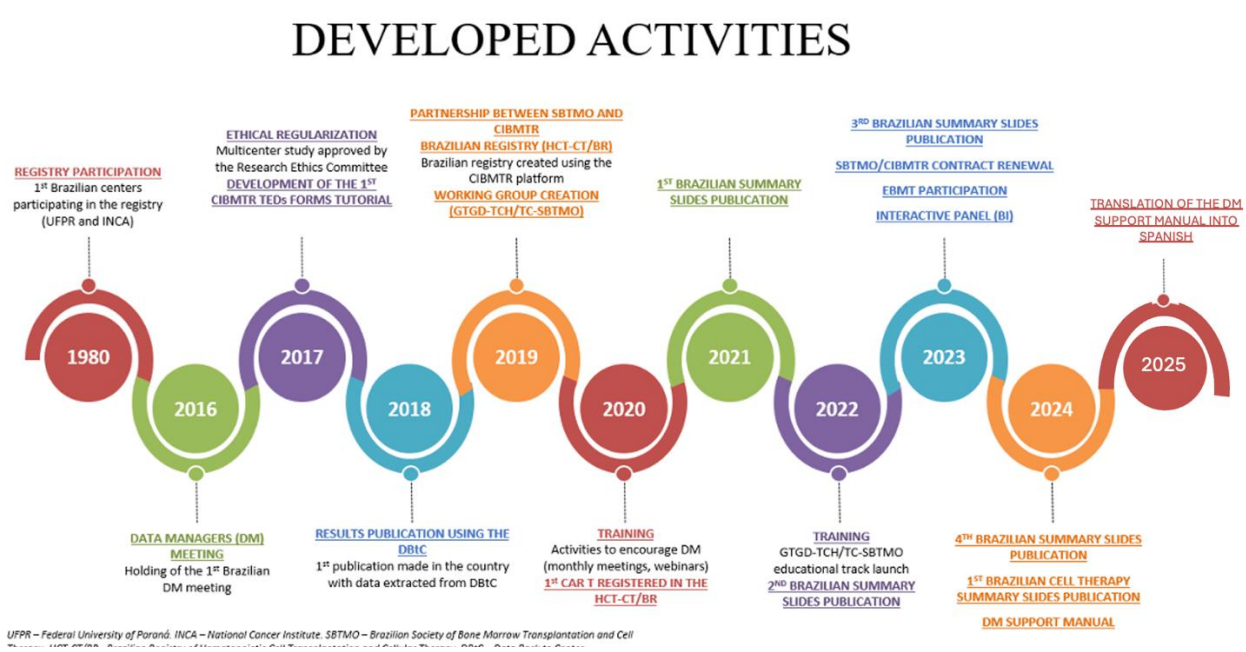

DEVELOPED ACTIVITIES
1980
REGISTRY PARTICIPATION
1st Brazilian centers participating in the registry (UFPR and INCA)
2016
DATA MANAGERS (DM) MEETING
Holding of the 1st Brazilian DM meeting
2017
ETHICAL REGULARIZATION
Multicenter study approved by the Research Ethics Committee
DEVELOPMENT OF THE 1ST CIBMTR TEDs FORMS TUTORIAL
2018
RESULTS PUBLICATION USING THE DBtC
1st publication made in the country with data extracted from DBtC
2019
PARTNERSHIP BETWEEN SBTMO AND CIBMTR
BRAZILIAN REGISTRY (HCT-CT/BR)
Brazilian registry created using the CIBMTR platform
WORKING GROUP CREATION (GTGD-TCH/TC-SBTMO)
2020
TRAINING
Activities to encourage DM (monthly meetings, webinars)
1st CAR T REGISTERED IN THE HCT-CT/BR
2021
1ST BRAZILIAN SUMMARY SLIDES PUBLICATION
2022
TRAINING
GTGD-TCH/TC-SBTMO educational track launch
2ND BRAZILIAN SUMMARY SLIDES PUBLICATION
2023
3RD BRAZILIAN SUMMARY SLIDES PUBLICATION
SBTMO/CIBMTR CONTRACT RENEWAL
EBMT PARTICIPATION
INTERACTIVE PANEL (BI)
2024
4TH BRAZILIAN SUMMARY SLIDES PUBLICATION
1ST BRAZILIAN CELL THERAPY SUMMARY SLIDES PUBLICATION
DM SUPPORT MANUAL
2025
TRANSLATION OF THE DM SUPPORT MANUAL INTO SPANISH
UFPR – Federal University of Paraná. INCA – National Cancer Institute. SBTMO – Brazilian Society of Bone Marrow Transplantation and Cell

FIGURE 2

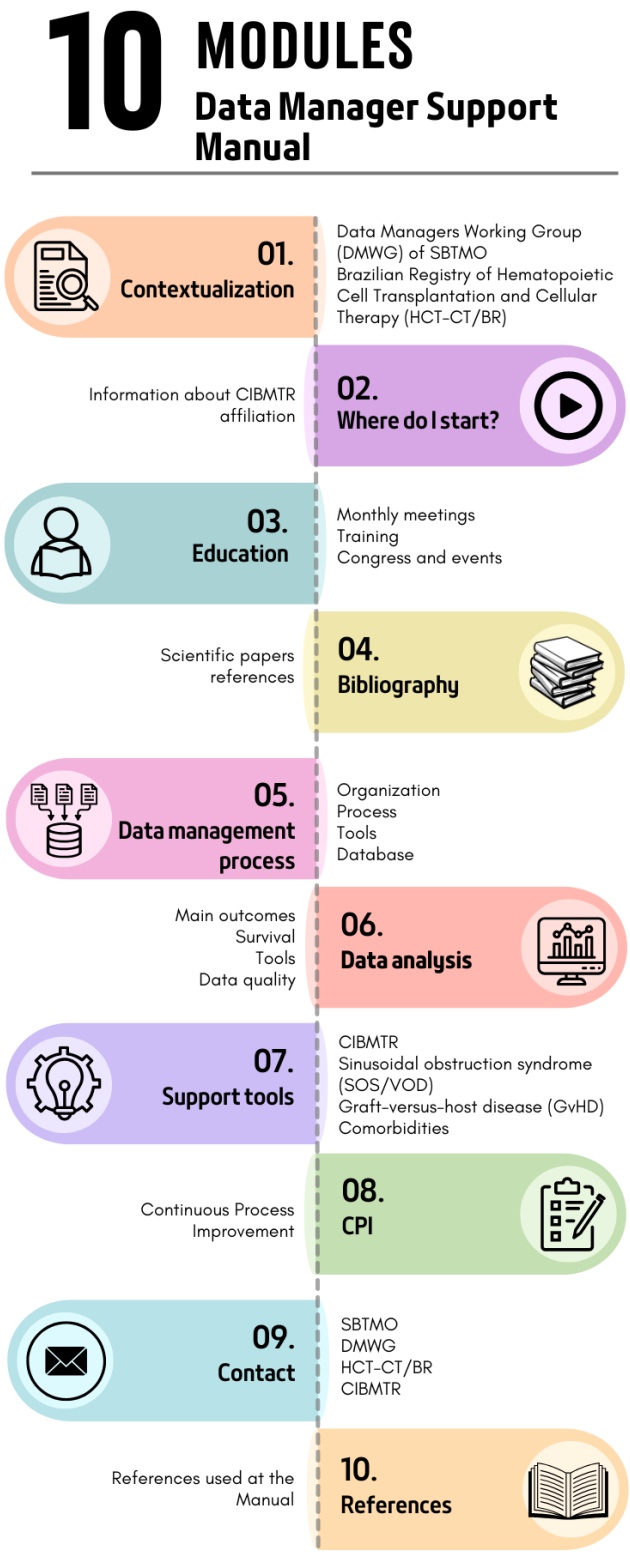

10 MODULES
Data Manager Support Manual
01. Contextualization
Data Managers Working Group (DMWG) of SBTMO
Brazilian Registry of Hematopoietic Cell Transplantation and Cellular Therapy (HCT-CT/BR)
Information about CIBMTR affiliation
02. Where do I start?
03. Education
Monthly meetings
Training
Congress and events
Scientific papers references
04. Bibliography
05. Data management process
Organization
Process
Tools
Database
Main outcomes
Survival
Tools
Data quality
06. Data analysis
07. Support tools
CIBMTR
Sinusoidal obstruction syndrome (SOS/VOD)
Graft-versus-host disease (GvHD)
Comorbidities
Continuous Process Improvement
08. CPI
09. Contact
SBTMO
DMWG
HCT-CT/BR
CIBMTR
References used at the Manual
10. References

FIGURE 3

FIGURE 4

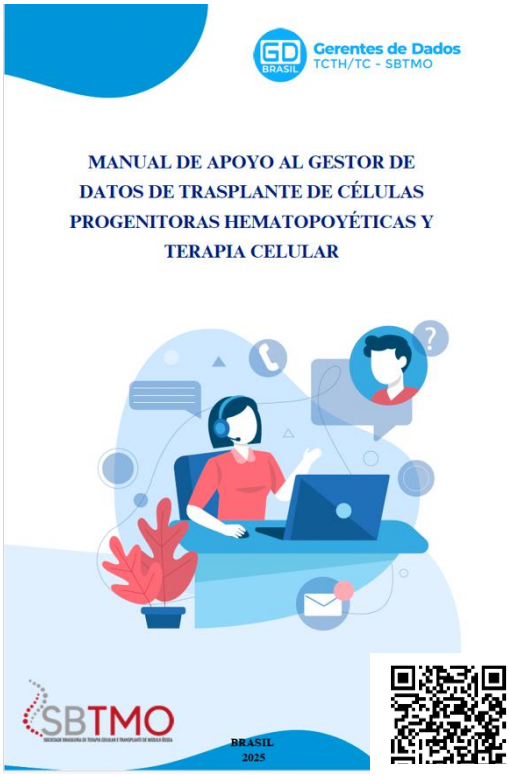
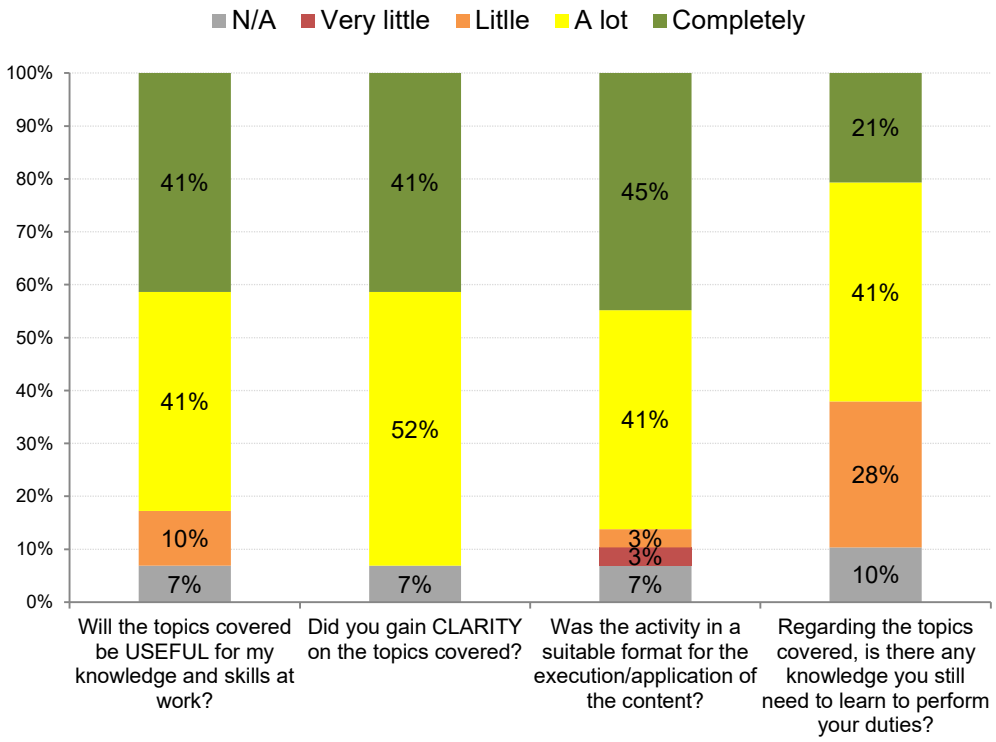


FIGURE 5


N/A
Very little
Litlle
A lot
Completely
100%
90%
80%
70%
60%
50%
40%
30%
20%
10%
0%
41%
41%
10%
7%
41%
52%
7%
45%
41%
3%
3%
7%
21%
41%
28%
10%
Will the topics covered be USEFUL for my knowledge and skills at work?
Did you gain CLARITY on the topics covered?
Was the activity in a suitable format for the execution/application of the content?
Regarding the topics covered, is there any knowledge you still need to learn to perform your duties?

ETHICAL AND LEGAL ASPECTS IN THE USE OF HEALTH DATA IN HSCT AND CELL THERAPY: PRACTICAL CONSIDERATIONS FOR DATA MANAGERS IN BRAZIL

Heliz Regina Alves das Neves[1]; Ana Paula Batistela[1]; Indianara Rotta[1]; Roseli R. Silva[1]; Adriana Mendes de Quadros Cavilha[1]; Luciana Nasser Dornelles[1]; Vanessa de Fatima Kukla[1]; Gisele Loth; Vaneuza Araújo Moreira Funke[1]; Samir Kanaan Nabhan[1]

1. Complexo Hospital de Clínicas Da UFPR, Curitiba - PR - Brasil.

INTRODUCTION:

With the consolidation of the Brazilian Registry of Hematopoietic Stem Cell Transplantation and Cellular Therapy (HSCT-TC), requests for patient data have become increasingly frequent — including some that are informally or improperly regulated. This scenario has created uncertainty among Data Managers (DMs), who often feel unprepared to respond appropriately. Brazil's General Data Protection Law (Lei Geral de Proteção de Dados – LGPD) offers a legal framework to guide the ethical and secure handling of personal and sensitive health data, such as those routinely collected in transplant and cellular therapy centers.

OBJECTIVE:

To present a practical experience of implementing LGPD principles in an HSCT center, clarifying legal boundaries for data sharing and reinforcing the technical and ethical role of Data Managers and transplant leaders.

METHODS:

This report describes a real-world approach taken by a transplant and cellular therapy team in response to increasing data demands. Data requests were categorized into four main types: 1) regulatory/ control body requests (e.g., health ministry, audits, accreditation entities); 2) research-related requests, with or without ethics approval; 3) internal institutional use (e.g., quality indicators, operational planning); 4) external demands, such as conference abstracts or scientific publications. Each category was reviewed according to applicable legal bases, including patient consent, public health interest, and ethics committee (CEP) approval. To ensure compliance and institutional oversight, data access requests were routed through the Education and Research Management Office (GEP), with the involvement of the Medical Division and, when applicable, the local Research Ethics Committee (CEP). A service desk request system (GLPI) was adopted to register and track data access demands, ensuring traceability, transparency, and alignment with internal policies (Figure 1).

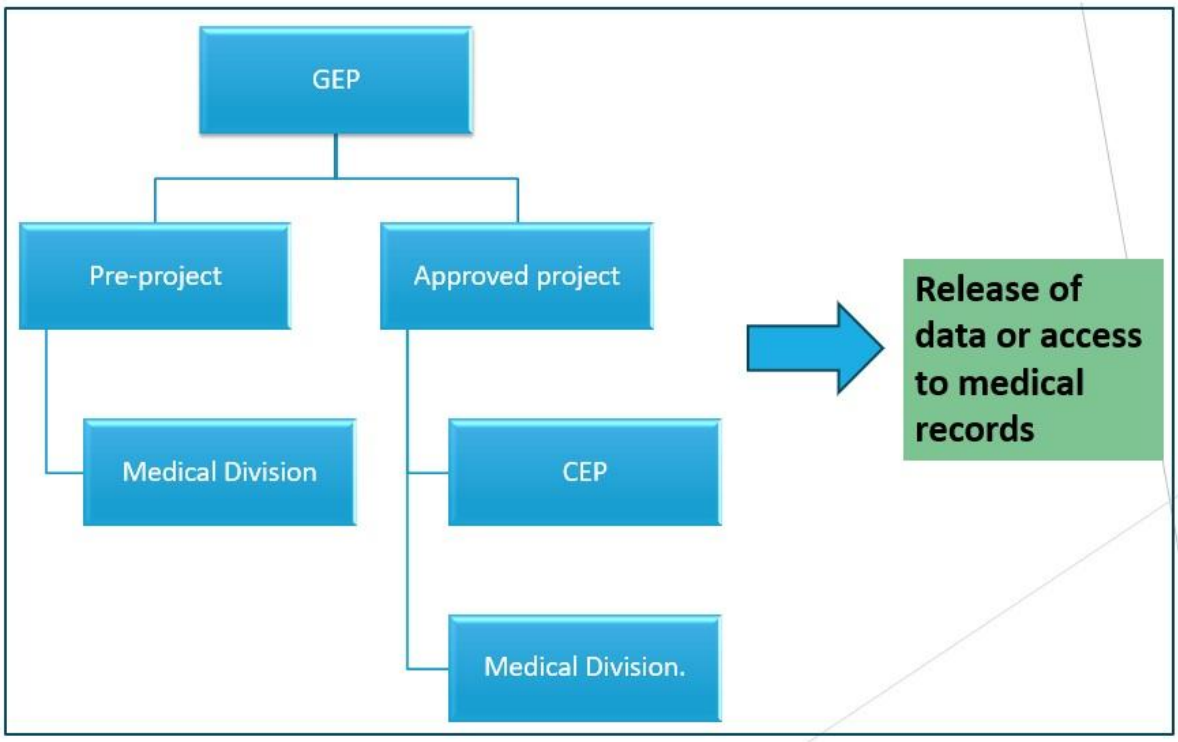
RESULTS:

The team established secure internal workflows grounded in LGPD principles and aligned with privacy rights guaranteed by the Brazilian Federal Constitution. It was reinforced that data submitted to national and international registries (e.g., SBTMO / CIBMTR, ABTO, LABMT) are under institutional responsibility and that local datasets must follow internal authorization flows. By distinguishing between appropriate and inappropriate uses, the team empowered DMs to act responsibly and with legal support. This initiative was presented and discussed during a monthly meeting of the SBTMO Data Managers' Working Group, providing a shared space for reflection and collective improvement.

CONCLUSION:

Beyond understanding the LGPD, HSCT-TC data professionals must be equipped with institutional tools, legal clarity, and well-defined operational protocols. This approach promotes ethical data sharing, safeguards patient rights, and strengthens the role of Data Managers within HSCT-TC governance.

Figure 1: Institutional workflow for handling data access requests in HSCT and Cell Therapy, ensuring compliance with LGPD and internal oversight.



EVALUATION OF ROBOTIC PROCESS AUTOMATION FOR DATA ENTRY IN THE CIBMTR FORMSNET3 PLATFORM

Rafael de Oliveira[1], Joaquim Gasparini dos Santos[1], Rafaela Picossi da Silva[1], Leonardo Jun Otuyama[1], Giancarlo Fatobene[1], Vanderson Rocha[1]

1. Laboratório de Investigação Médica (LIM) 31, Serviço de Hematologia, Hemoterapia e Terapia Celular, Hospital das Clínicas HCFMUSP, Faculdade de Medicina, Universidade de São Paulo, Brazil.

INTRODUCTION:

Accurate and timely reporting of clinical data to the Center for International Blood and Marrow Transplant Research (CIBMTR) is essential for outcomes monitoring and compliance in hematopoietic stem transplantation (HCT). FormsNet3 is the primary interface for submitting such data, but manual entry remains labor-intensive, error-prone, and highly variable. Robotic Process Automation (RPA) offers a scalable solution for automating repetitive data entry tasks with increased speed and consistency. This study evaluates the accuracy and efficiency of RPA in completing CIBMTR FormsNet3 entries, compared to manual processes, using real data from patients undergoing autologous HCT.

OBJECTIVE:

To compare the efficiency and accuracy of manual versus automated data entry in CIBMTR FormsNet3.

METHODS:

We assessed data entry time (in seconds) for two scenarios in FormsNet3 system: 'new entry' (CRID assignment F2804, Consent Tool, F2814, and F2400) and 'post-transplant reporting' (F2450), for autologous HCT. Data entry was executed on a laptop (Intel Core i7-12700H processor, 16 GB RAM, 1 TB SSD) with wired internet connection (average speed: download 94.8 Mb/s; upload: 96.4 Mb/s). Google Chrome v135 was used as the browser. Manual versus automated entry was compared. Manual data entry was performed by three data managers using dual-monitor setups for real-time data consultation. Automated entry utilized Python scripts with the Selenium library (v4.28.1). All data were preloaded into a REDCap database, accessible to both data managers and automation algorithms. Entry errors during data completion were quantified. Mean entry times, standard deviations, and entry error rates were calculated. Comparisons between manual and automated times were performed using the Wilcoxon signed-rank test.

RESULTS:

In the 'new entry' scenario, each method completed data entry for six patients. Mean entry time was 487 ± 71 seconds for manual entry compared to 209 ± 23 seconds for automated entry, representing a 42% reduction in time ($p = 0.03$) (Figure 1A). In the 'post-transplant reporting' scenario, each method completed 36 patient forms. Mean entry time was 144 ± 43 seconds for manual entry compared to 45 ± 10 seconds for automated entry, a 31% time reduction ($p < 0.001$) (Figure 1B). Manual entry errors were found on one patient (17%) in the 'new entry' scenario, and in 5 forms (14%) for the 'post-transplant reporting' scenario. No errors were observed in the automated entries.

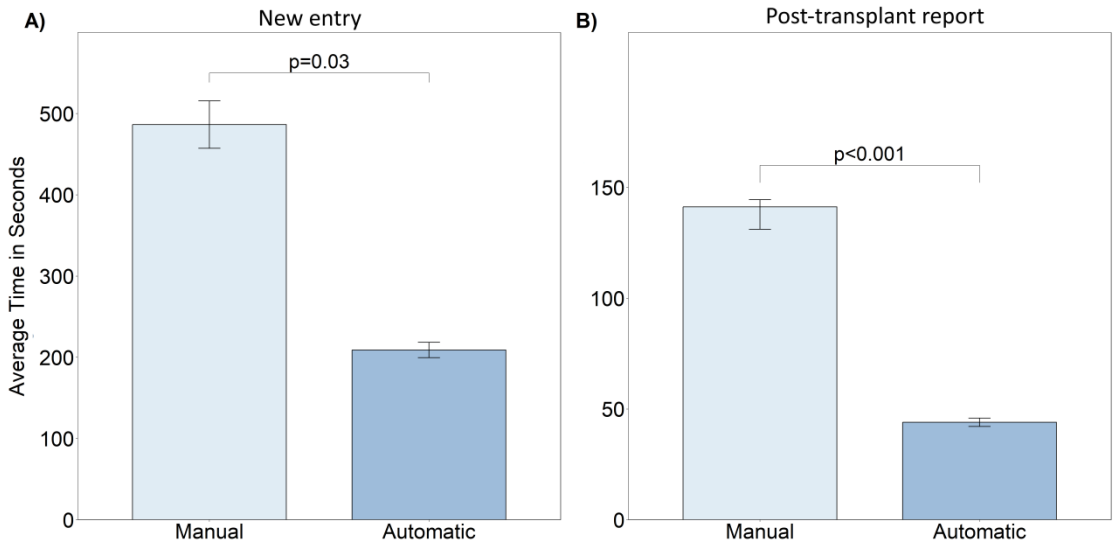
CONCLUSION:

RPA significantly reduced the time required for data entry into CIBMTR FormsNet3 in both scenarios, while also eliminating data entry errors. Manual entry was associated with a notable error rate. These findings support the use of RPA as a reliable and efficient tool in clinical data workflows, enabling data managers to focus more on data validation and analysis tasks.

KEYWORDS:

CIBMTR FormsNet3, Data Entry, Robotic Process Automation.

FIGURE 1: Comparison of manual and automated methods for new patient registration (A) and follow-up data entry (B).



HISTORICAL ANALYSIS OF THE BRAZILIAN REGISTRY OF VOLUNTARY BONE MARROW DONORS AND ITS ROLE IN FACILITATING CELLULAR PRODUCTS FOR BRAZILIAN PATIENTS

Natiele Tavares[1], Pamela Lopes1, Andrea Copello[1], Renata Esterque[1], Adriana Nunes[1], Sheila Prado[1], Danielli Oliveira[1]

1 – Registro Brasileiro de Doadores Voluntários de Medula Óssea (REDOME) – Ministério da Saúde

INTRODUCTION

The Bone Marrow Donor Registry (REDOME) has played a crucial role in connecting donors and patients in need of transplants since 1993. However, over the past ten years, there has been a significant increase in the number of cellular products made available, reflecting the effectiveness of established processes, the competence of the team, and management's ability to continuously improve outcomes despite the many challenges and complexities of the program. The program is linked to the Ministry of Health through the National Transplant System (SNT), with technical management by the National Cancer Institute (INCA). This study aims to analyze the historical series from 2014 to 2024, highlighting 2024 as the year with the highest number of products made available by the program, demonstrating REDOME's growth and ongoing importance for patients and society as a whole.

OBJECTIVE

The objective of this study is to evaluate the evolution of cellular products provided to Brazilian patients from national and international donors, facilitated by REDOME between 2014 and 2024, identifying trends and annual variations, with particular emphasis on 2024, as it was the highest in the historical series.

METHOD

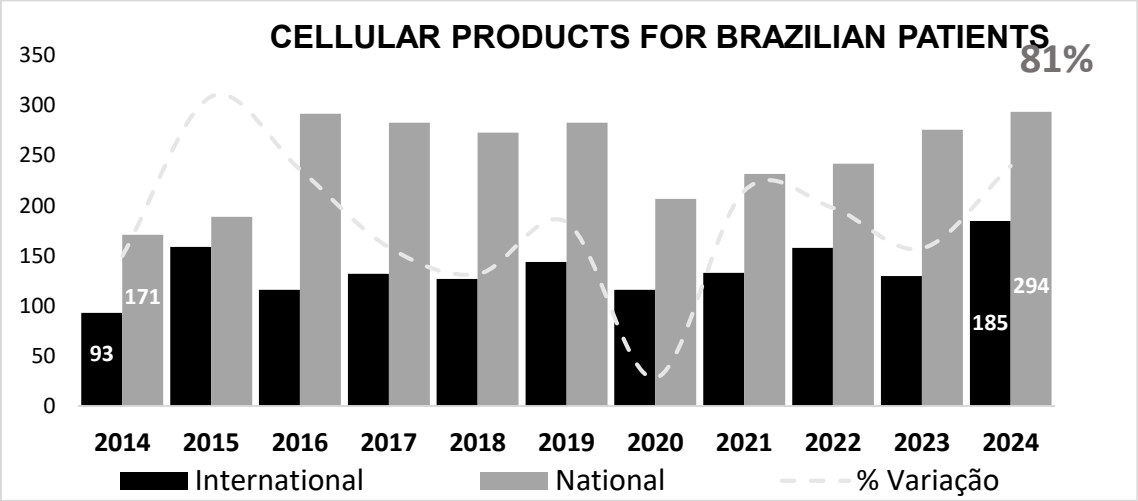
A descriptive analysis was conducted on annual data of cellular products made available by REDOME from 2014 to 2024. The study focused on year-to-year comparisons, emphasizing the growth in collections of cellular products from both national and international donors.

RESULTS

From 2014 to 2024, there was an 81% increase in the total number of cellular products made available. Regarding donation sources, in 2014, 94% of collections were from bone marrow and 0% from peripheral blood, whereas in 2024, these proportions shifted to 26% bone marrow and 70% peripheral blood. In the context of national donors, 2024 stood out with 294 products, whereas the previous peaks were in 2016 (295) and 2019 (290). From 2014 to 2024, there was a 72% increase. As for international donors, REDOME reached its highest number in 2024 (185), followed by 2015 (159) and 2022 (158). Also between 2014 and 2024, there was a 99% increase.

CONCLUSION

The findings indicate that 2024 was the year in which REDOME provided the highest number of cellular products for Brazilian patients in its entire historical series. Despite the immense challenges posed by such a complex operation, the commitment of management, the team, and the entire network of collection centers, transplant units, laboratories, and other institutions involved in each step of the process was crucial. Above all, the key element that made this historical series successful was the donors; without them, none of this would have been possible.



	BM	PBSC	CBU	DLI	Total
2014	251	0	14	3	268
International	82	0	10	1	93
National	169	0	4	2	175
2015	321	0	16	13	350
International	144	0	14	1	159
National	177	0	2	12	191
2016	355	26	12	18	411
International	97	1	9	9	116
National	258	25	3	9	295
2017	293	92	14	20	419
International	118	0	10	4	132
National	175	92	4	16	287
2018	225	151	9	16	401
International	71	46	8	2	127
National	154	105	1	14	274
2019	248	150	17	19	434
International	66	66	10	2	144
National	182	84	7	17	290
2020	163	139	10	15	327
International	53	53	6	4	116
National	110	86	4	11	211
2021	136	207	10	13	366
International	35	85	9	4	133
National	101	122	1	9	233
2022	159	223	5	14	401
International	50	99	4	5	158
National	109	124	1	9	243
2023	127	266	4	9	406
International	33	89	4	4	130
National	94	177	0	5	276
2024	126	334	6	13	479
International	38	139	5	3	185
National	88	195	1	10	294
Total	2404	1588	117	153	4262

IMPLEMENTATION OF CLINICAL TEMPLATES IN HEMATOPOIETIC CELL TRANSPLANTATION: STANDARDIZATION OF MEDICAL PROGRESS NOTES AND IMPROVEMENT IN DATA COLLECTION

Jessica Di Chiara Salgado[1], Luiz Carlos da Costa-Junior[1], Marina Izu[1], Simone Pereira Lermontov[1], Valéria Gonçalves[1], Thayane Vicente de Aquino[1], Simone Cunha Maradei[1], Marta Colares[1], Maria Claudia Rodrigues[1], Patrícia Regina Cavalcanti Barbosa Horn[1], Decio Lerner[1]

1. Bone Marrow Transplant Center, Instituto Nacional de Câncer (INCA), Rio de Janeiro, RJ, Brasil

INTRODUCTION:

The fragmentation of information in medical progress notes hinders data collection, reporting activities, and integrated care. The quality of medical records for patients undergoing hematopoietic cell transplantation (HCT) is essential for proper clinical follow-up. Additionally, it directly impacts data reporting to national and international registries, such as the Brazilian Transplant Registry and the Center for International Blood and Marrow Transplant Research (CIBMTR). To address this gap and the lack of critical data, a standardization tool was developed to improve data completeness and accuracy, aiming to optimize the work of data managers.

OBJECTIVE:

To describe the development process of a tool composed of standardized templates, with the goal of improving clinical documentation, increasing efficiency in the documentation process, and enhancing the quality of reported data in the context of HCT.

METHODS:

This is a methodological study focused on the development and implementation of a structured tool to standardize clinical documentation. Three templates were created based on the institutional model of the University of São Paulo (USP) and validated by a multidisciplinary team: (1) Unified pre-HCT template by disease, including date/diagnostic methodology, Karnofsky/Lansky score and disease status; (2) Discharge Summary with essential data for outpatient follow-up; (3) Post-HCT (D+100/180/365) with forms for engraftment, GVHD (MAGIC/NIH criteria), infections, and therapy. The templates were developed in alignment with the forms and requirements of the CIBMTR FormsNet3 platform for consultations and strategic time points such as pre-transplant assessment, discharge summary, and post-transplant evaluations.

RESULTS:

The implementation of the templates enabled the centralization of critical clinical information, with fields mapped directly to the CIBMTR's FormsNet3 platform (e.g., chimerism data, GVHD prophylaxis), and standardized key definitions, such as neutrophil engraftment (absolute neutrophil count >500/mm^3 for 3 consecutive days). It is expected that adopting the tool will improve the completeness of recorded information, especially in essential variables such as disease status at the time of HCT, and post-transplant infectious or GVHD-related events. Furthermore, a reduction is anticipated in the time required to retrieve and record clinical data. In a subsequent phase of the study, a quantitative analysis will be conducted to compare data completeness before and after template implementation to evaluate the direct impact of standardization on clinical documentation quality.

CONCLUSIONS:

The use of clinical templates proved to be a strategy for standardizing HCT records, improving data quality, and facilitating reporting to the CIBMTR. Next steps include expanding to other centers and assessing the impact on data completeness.

KEYWORDS: standardization, data quality, bone marrow transplantation.

FIGURE 1. Pre-HCT Assessment Template – AAS

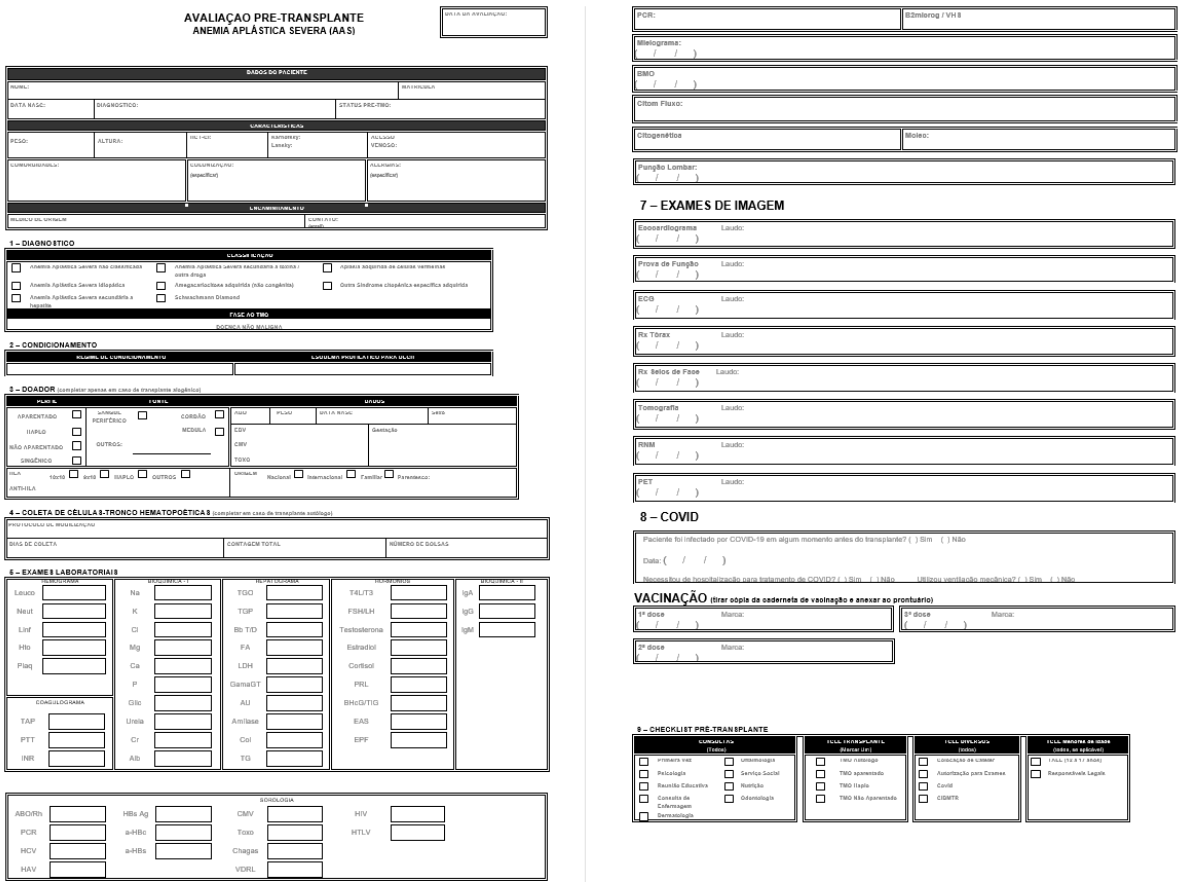


FIGURE 2. Allogeneic Post-HCT Assessment Template

Avaliação Pós-TMO Alogênico
(D+100, D+180, D+365)

Nome
Idade
Serviço de origem

1) D+X TCH Alogênico (Tipo de doador e produto)
- Indicação (doença e status pré-transplante – informação objetiva)
- Regime de condicionamento (Intensidade do condicionamento e drogas/dose)

Mieloablativo, RIC e não-mieloablativo
- Informações do doador (idade, sexo, parentesco, ABO, sorologia CMV e compatibilidade HLA) (10x10, 9x10, 5x10)
- Data, fonte de células infundidas
- Usos de fatores de crescimento (sim ou não, e especificar, se possível)
- Profilaxia DECH
  - CSA
  - Tacrolimus (FK)
  - MMF
  - MTX
  - PTCy

Em uso de alguma profilaxia no momento? Qual?

- Profilaxia SOS/hepática: Não Sim Qual (Ursacol, outro)
- Enxertia
  - Pega neutrofílica – Data (primeiro de 3 dias consecutivos com N > 500/mm3)
  - Pega plaquetária – Data (primeiro de 7 dias consecutivos com Plaq > 20.000/mm3)

O paciente ainda tem necessidade de terapia transfusional? (sim ou não)
Concentrado de plaquetas Concentrado de hemácias
Data da última transfusão

2) Infeccioso:
- Sorologias do receptor e doador (CMV)
- Profilaxia infecciosa e data de início
  Aciclovir, Bactrim, antifúngico (especificar), outros
- Intercorrências infecciosas:
  - Especificar tipo de infecção (bacteriana, fúngica, viral, outros)
  - Data
  - Diagnóstico clínico (agente e manifestação clínica)
  - Tratamento

3) Quimerismo:
- Método (VNTR)
- SP ou MO
- Percentual

4) Status hematológico atual:
- Hemograma, necessidade transfusional, sobrecarga de ferro

5) DECH Aguda: Formulário em anexo
- Diagnóstico
- Data do diagnóstico
- Classificar de acordo com os critérios *Magic*
  - Global, pele, TGI superior e inferior, fígado
    - Ao diagnóstico (global e data – por órgãos somente no registro do diagnóstico)
    - Grau máximo (global e data)
- Tratamento instituído – especificar datas

6) Exame físico:
- Sempre especificar:
  - ECOG/KPS
  - Altura e peso (no início do seguimento e atual)

7) DECH Crônica: Formulário em anexo
- Diagnóstico (de novo, progressivo, interrompido ou *overlap*)
- Data do diagnóstico
- ECOG/KPS ao diagnóstico
- Escore NIH
  - Global:
    - Ao diagnóstico (detalhado no registro de diagnóstico)
    - Máximo (data somente se limitada ou extenso/leve a grave)
    - Atual (detalhado)
  - Por órgão acometido (Escore 0-3)
    - Pele (considerar uso do Rodnan modificado)
    - Boca
    - Olhos
    - TGI
    - Fígado
    - Pulmões
    - Articulações e fáscias (considerar uso do P-ROM)
    - TGU
  - Tratamento instituído (inicial e sequencial):

8) Outras complicações:
- Não relacionadas ao regime de condicionamento ou DECH e não infecciosas

9) Tratamentos realizados após o TCH Alogênico:
- Infusão de linfócitos do doador, ITKs, Hipometilante, etc;
- Especificar se eram intervenções programadas pré-TCH ou não, assim como o motivo

10) Status atual da doença de base:
- Remissão completa, recidiva (data da última avaliação)

Referente à data da última avaliação:
( ) Continuou em Resposta Completa
( ) Resposta Completa
( ) Não Alcançou Resposta Completa
( ) Não avaliado
( ) Doença Detectada
( ) Doença não detectada, mas avaliação insuficiente para estabelecer RC
( ) Resposta parcial
( ) Doença estável
( ) Progressão da doença

11) Medicações em uso:
12) Evolução:
13) Exame físico:
- Sempre especificar
  - ECOG/KPS
  - Altura e peso (no início do seguimento e atual)

14) Últimos exames:
Laboratório e imagem pertinentes
15) Impressão clínica e programação:
16) Conduta:

INTERNAL AUDIT AS A PILLAR FOR TRAINING AND STANDARDIZATION OF DATA QUALITY IN HEMATOPOIETIC CELL TRANSPLANTATION (HCT)

Anderson João Simione[1], Bruna Fernanda Sona Mathias[1], Jaine Cristina de Oliveira Silva[1], Iago Colturato[1], Aline Saggioro Vieira[1], Aline Maglim Gonçalves de Oliveira Godoy[1], Ana Claudia Ferrari dos Santos[1], Valquiria de Cassia Possani[1], Fernanda Rodrigues Barbieri[1], Erika Rodrigues Pontes Delattre[1], Anna Beatriz Coelho de Souza[1], Carolina Ferreira Mascarenhas[1], Gessica Augusto[1], Mair Pedro de Souza[1], Vergilio Antonio Rensi Colturato[1]

1 Hospital Amaral Carvalho, Jaú, Brazil

INTRODUCTION

The accuracy and standardization of clinical data are essential for evaluating outcomes in hematopoietic cell transplantation (HCT), especially in multicenter research and quality management. In this context, internal audits function as strategic tools for quality control, enabling the identification of inconsistencies. When integrated into training processes, they support the practical education of data management teams, facilitating the understanding of clinical definitions and alignment with standardized reporting protocols.

OBJECTIVE

To demonstrate the role of internal audits as a training tool for data management teams in HCT, highlighting their contribution to identifying inconsistencies and improving the quality of clinical information.

METHODS

The internal audit was conducted from September 16 to 20, 2024, following CIBMTR guidelines. Ten patients who underwent HCT between 2020 and 2023 were randomly selected. Transplant Essential Data (TED) forms were audited up to one year post-transplant, totaling 1,192 fields (964 critical, 228 random). The audit was performed by the center's data manager, focusing on identifying inconsistencies and training the team.

RESULTS

A total of 1,192 fields were audited, with an overall error rate of 5.37%. Of these, 28.13% were related to post-transplant disease status, especially best response and current status. Most of these errors occurred in multiple myeloma patients, where therapeutic response assessment is more complex due to varied disease presentations and the need for multiple specific tests. Not all centers have timely access to these tests, which may hinder the confirmation of complete response. In response, a targeted action plan was developed, including two standardized workflows based on CIBMTR guidelines to guide the classification of best response (Figure 1) and current disease status (Figure 2). These workflows were implemented as training tools, enhancing understanding of clinical criteria and reinforcing data standardization. Following this, a systematic review of 263 transplants (2020–2024) in multiple myeloma patients was initiated. Among these, 106 cases (40.3%) required corrections in at least one of the submitted forms, particularly regarding disease status variables.

CONCLUSION

Internal auditing proved to be an effective strategy for improving data quality in HCT, enabling the identification of inconsistencies and guiding corrective actions. The implementation of standardized workflows strengthened the team's technical skills and promoted greater alignment with clinical definitions. The review of 263 cases resulted in significant data corrections, confirming the practical value of the intervention. Moving forward, regular audits and continuous training will be essential to ensure the accuracy and reliability of information submitted to the international registry.

FIGURE 1. Workflow for classifying the best response to transplant in patients with multiple myeloma.

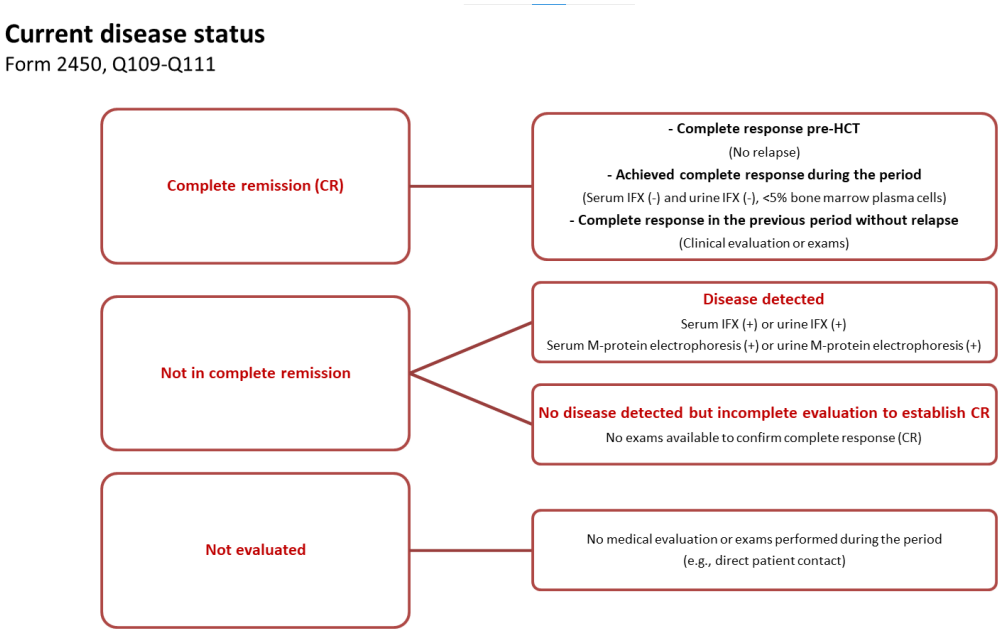
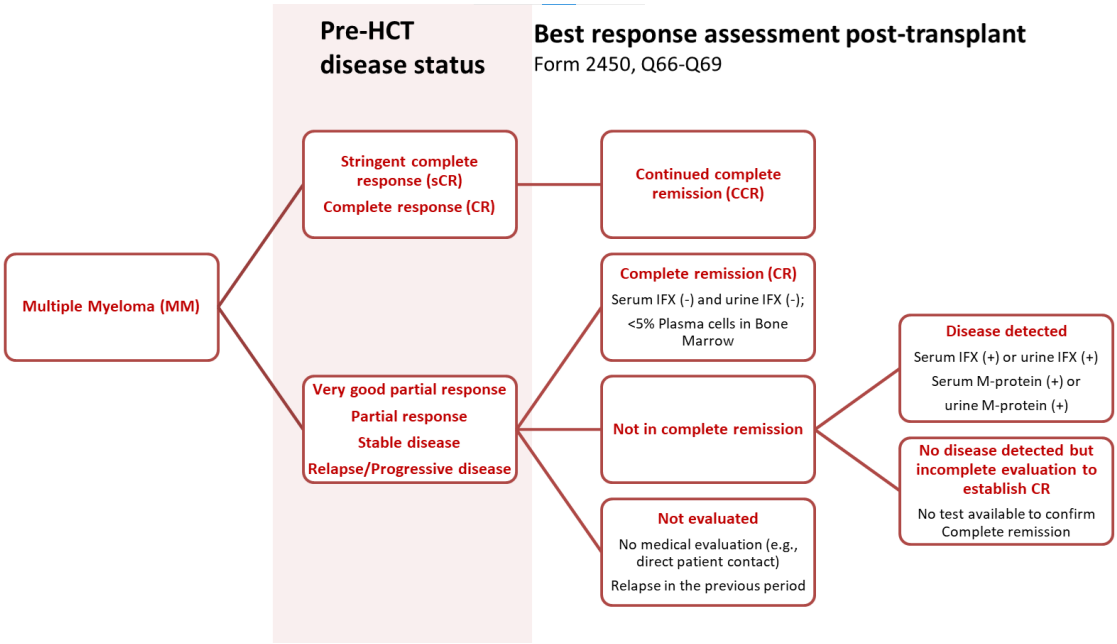


FIGURE 2. Workflow for classifying current disease status in patients with multiple myeloma.



MAPPING BRAZILIAN SCIENTIFIC ABSTRACTS ON DATA MANAGEMENT IN HEMATOPOIETIC CELL TRANSPLANTATION CONFERENCES: AN ANALYSIS OF INTERNATIONAL AND NATIONAL CONTRIBUTIONS

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INTRODUCTION:

In the hematopoietic cell transplantation (HCT) scenario, the systematic management of clinical data and the dissemination of research findings through abstracts are crucial for advancing clinical practices and improving patient outcomes. Effective data management (DM) not only ensures the accuracy and reliability of research but also enhances the ability to assess trends and outcomes.

OBJECTIVE:

To assess the Brazilian scientific production trough number of abstracts published in main scientific HCT meetings in the field of data management compared to other countries.

METHODS:

A bibliometric study was conducted to identify meeting abstracts published in Transplantation and

Cellular Therapy (TANDEM meetings), Bone Marrow Transplantation (EBMT) and Journal of Bone Marrow Transplantation and Cellular Therapy (JBMTCT - SBTMO), in the DM category from 2017 to 2024. They were classified according to the first author continent of origin, except for Brazil and the USA since they were the only country from South and North America respectively. Publications dates were classified into triennia, except for 2023 and 2024. The EBMT 2024 abstract book was not available at the time of this abstract. Abstracts presented at SBTMO were considered from 2020 onwards due to JBMTCT being available since that date.

RESULTS:

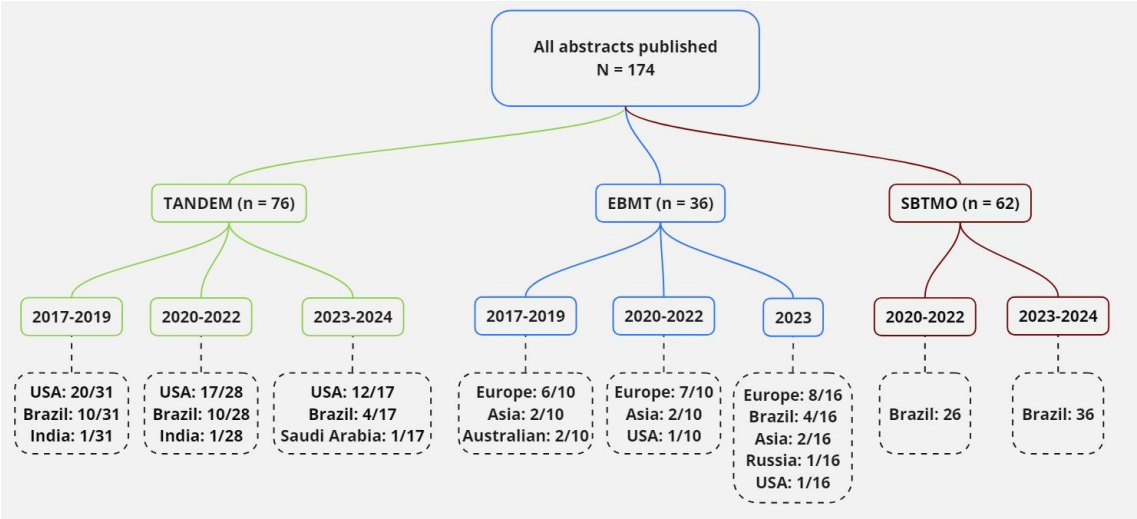
It was identified 183 abstracts submitted to the conference's DM category. However, 5 were excluded because the contents were not related to the subject, resulting in 178 abstracts. From these, 43% were presented at the TANDEM meetings from 2017 to 2024, 19% at EBMT from 2017 to 2023 and 38% at SBTMO from 2020 to 2024 (Figure 1). Abstracts from Brazil accounted for 10 (34%) of the 29 abstracts presented at tandem in the 2017-2019 triennium, and for 11 (37%) of the 30 in the next triennium. However, none were presented at EBMT at the same period. In the last period analysed, Brazil was responsible for 4 (22%) abstracts from 18 at TANDEM, as well as 4 (33%) from 12 at EBMT. It was observed that the USA had consistently high numbers of abstracts presented at TANDEM, but lower number at EBMT. In contrast, European countries have higher contribution to EBMT than to TANDEM. In Brazil, the main HCT conference is the SBTMO meeting which had all abstracts presented by Brazilian data managers, totalling 68 abstracts between 2020 and 2024.

CONCLUSION:

Brazilian data managers scientific production as abstracts has always been consistent at TANDEM, and more recently at EBMT, but the largest number of abstracts is still concentrated at the main national HCT conference (SBTMO). It was observed that countries have tendency to present abstracts at conferences in their region, possible due to logistical and financial reasons.

KEYWORDS: Data Management; Scientific Production; Hematopoietic Cell Transplantation

FIGURE 1. Abstracts presented at TANDEM, EBMT and SBTMO in the data management category.



OPERATIONAL, QUALITY, AND SAFETY DIFFERENCES BETWEEN SPONSORED AND INVESTIGATOR-INITIATED CLINICAL TRIALS: A SINGLE-CENTER COMPARATIVE ANALYSIS

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INTRODUCTION:

Randomized clinical trials (RCTs) are essential for validating therapeutic interventions. However, industry-sponsored trials (SPON) and investigator-initiated trials (IITs) may differ substantially in operational, regulatory, and quality-related aspects. Understanding these differences is crucial for optimizing institutional research workflows and strengthening clinical trial governance.

OBJECTIVE:

To compare operational, recruitment, quality, and safety indicators between sponsored and investigator-initiated RCTs conducted at a single clinical research center.

METHODS:

This was a descriptive, retrospective study evaluating four active randomized controlled trials (RCTs) conducted between 2023 and 2024, comprising two industry-sponsored trials (SPON) and two investigator-initiated trials (IITs). A total of 68 patients were under active follow-up during the study period, including 6 (8.8%) enrolled in SPON trials and 62 (91.2%) in IITs. Twelve predefined performance indicators were assessed across four domains: (1) Operational – mean time from ethics submission to approval, and from approval to recruitment start; (2) Recruitment – inclusion rate and screening failure rate; (3) Quality – number of internal audits, monitoring visits, team training sessions, and availability of backup staff; and (4) Safety – number of adverse events (AEs), serious adverse events (SAEs), and protocol deviations. Mean values per trial were calculated for each group and presented in a comparative heatmap (Figure 1).

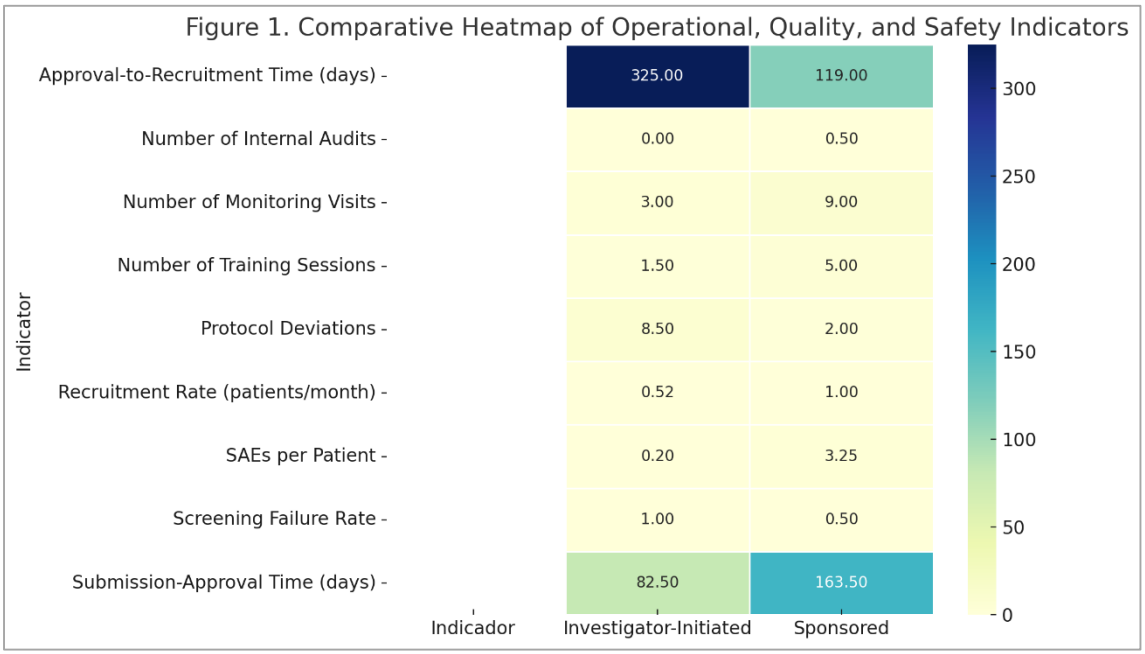
RESULTS:

Each group represented 50% of the active RCTs. The mean time from submission to ethics approval was higher in SPON trials (163.5 days) compared to IITs (82.5 days). In contrast, the time from approval to recruitment initiation was shorter in SPON (119 vs. 325 days). SPON trials also had a higher recruitment rate (0.995 vs. 0.515 patients/month) and a lower screening failure rate (0.5 vs. 1.0). Regarding quality indicators, SPON trials had more internal audits (0.5 vs. 0), monitoring visits (9.0 vs. 3.0), and internal training sessions (5.0 vs. 1.5). All studies had trained backup staff available. In the safety domain, SPON trials reported more SAEs per patient (3.25 vs. 0.2), as well as more AEs and protocol deviations. Over the past 12 months, most research proposals submitted to the institutional review office originated from IITs. Consolidated comparative results are presented in Figure 1 (heatmap).

CONCLUSION:

Sponsored RCTs showed superior performance across operational, quality, and safety indicators, reflecting greater technical support and regulatory structure. Conversely, IITs demonstrated faster initial ethics processing and greater scientific productivity. These findings underscore the need for tailored support strategies to strengthen both clinical trial models within institutional settings.

FIGURE 1. Heatmap comparing mean indicators between sponsored and investigator-initiated RCTs.



OUTCOMES OF HEMATOPOIETIC PROGENITOR CELL MOBILIZATION AND COLLECTION IN CHILDREN: A RETROSPECTIVE ANALYSIS

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INTRODUCTION:

The mobilization and collection of hematopoietic progenitor cells (HPC) are critical steps in preparing for bone marrow transplantation (BMT), particularly in pediatrics. The success of this process directly affect the engraftment and clinical outcomes. The Hospital da Criança de Brasília José Alencar (HCB) has consolidated its autologous BMT service in recent years, achieving increasingly positive results in pediatric patients.

OBJECTIVE:

To evaluate outcome indicators related to hematopoietic progenitor cells mobilization and collection in children undergoing autologous BMT, including mobilization efficacy, time between collection and transplantation, and clinical progression.

METHODS:

A retrospective, descriptive study conducted at the HCB BMT Unit between September 2019 and May 2024. Data were extracted from the unit's management database. Fifty-seven pediatric patients who underwent hematopoietic progenitor cells mobilization and collection for autologous BMT were analyzed. Variables assessed included diagnosis, mobilization regimen, number of apheresis sessions, CD34+ cell yield, time from collection to BMT, engraftment time, and post-transplant status.

RESULTS:

Most frequent diagnoses: Neuroblastoma (47%), Germ Cell Tumors (16%), and Hodgkin's Lymphoma (11%). Mobilization regimens used: Chemotherapy + G-CSF (63%), G-CSF alone (26%), G-CSF + Plerixafor (5%), and Chemotherapy + G-CSF + Plerixafor (5%). The mean CD34+ cell yield was 101 $\times 10^6$/kg, with a mean infused dose of 90 $\times 10^6$/kg. The average number of apheresis sessions per patient was 1. The successful mobilization rate was 93%.The median time from collection to transplantation was 67 days (mean: 105 days; range: 8–630 days). The mean time to neutrophil engraftment was 14 days. The post-BMT mortality rate was 11%.

CONCLUSION:

The hematopoietic progenitor cells mobilization and collection in children demonstrated high efficacy, low need for multiple collections, good tolerability, and positive clinical outcomes. The variable interval between collection and BMT reflects individualized case management. These findings reinforce the feasibility and safety of HPC mobilization in pediatric BMT.

KEYWORDS: Bone Marrow Transplantation, Pediatrics, Health Services Epidemiology.

PATIENT-REPORTED OUTCOMES FOLLOWING HEMATOPOIETIC CELL TRANSPLANTATION

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INTRODUCTION:

Significant advances in hematopoietic stem cell transplantation (HSCT) have resulted in improved survival and cure rates. However, these outcomes do not necessarily correlate with patient satisfaction or quality of life (QoL). Currently, multiple validated tools are available to assess patient-reported outcomes and their perception of QoL during different phases of the transplant process.

OBJECTIVE:

This was a single-center, prospective study designed to assess the QoL of patients undergoing HSCT using the translated and validated FACT-BMT questionnaire, which covers multiple QoL domains.

METHODS:

Patients undergoing autologous or allogeneic HSCT at Hospital Brasília between 2021 and 2023 were evaluated using the FACT-BMT tool. The questionnaire was applied before transplantation and again on day +30 post-transplant. The Wilcoxon Signed-Rank Test was used for statistical analysis.

RESULTS:

A total of 37 patients were evaluated, with a median age of 59 years (Autologous HSCT: n=16; Allogeneic HSCT: n=21). The most frequent underlying diseases were multiple myeloma (43%) and acute myeloid leukemia (22%). Four domains were assessed: physical well-being, social/family well-being, emotional well-being, and functional well-being. Comparison between pre-transplant and day +30 scores revealed a non-significant trend toward improvement in the first three domains. However, a statistically significant decline in functional well-being was observed post-transplant (pre-HSCT: 19.34 vs. D+30: 17.72; p=0.040). Functional well-being was found to be the most significantly impacted domain (17.54 vs. 7.92; p<0.01).

CONCLUSION:

This study highlights a significant decline in functional well-being on day +30 post-HSCT, emphasizing the need for targeted interventions to improve patient satisfaction during hospitalization. Recommendations include adapting light administrative work for remote execution, enhancing psychological support with appropriate expectation management, optimizing symptom control (especially sleep hygiene), and encouraging engagement in pleasurable activities such as occupational therapy and hobbies.

KEYWORDS:

quality of life, hematopoietic stem cell transplantation, FACT-BMT

	Pre-Transplant	D+30	Comparison
	n= 37	n=36	n=36
Physical Well-Being	18.06 (6.42)	18.86 (5.79)	0.454
Social/Family Well-Being	10.96 (4.14)	11.28 (4.20)	0.706
Emotional Well-Being	17.96 (3.35)	18.44 (2.87)	0.603
BemFunctional Well-Being	19.34 (5.71)	17.72 (5.49)	0.040

PROPOSAL OF A SEMI-AUTOMATED METHODOLOGY FOR AUDITING PATIENT DATA REGISTERED IN THE CIBMTR PLATFORM

Heliz Regina Alves das Neves[1]; Indianara Rotta[1]; Roseli R. Silva[1]; Adriana Mendes de Quadros Cavilha[1]; Luciana Nasser Dornelles[1]; Vanessa de Fatima Kukla[1]; Gisele Loth[1]; Vaneuza Araújo Moreira Funke[1]; Samir Kanaan Nabhan[1]

1. Complexo Hospital de Clínicas da UFPR, Curitiba - PR - Brasil.

INTRODUCTION:

Large transplant registries like the CIBMTR are essential for research and clinical benchmarking. However, the accuracy of submitted data, especially regarding diagnoses, conditioning, and follow-up, remains a challenge due to the complexity of hematopoietic stem cell transplantation (HSCT) cases. Objective: To propose a structured methodology for auditing CIBMTR data submissions, identify common sources of errors, and develop a reproducible workflow to support data managers (DM).

METHODS:

A manual audit was developed using predefined quality checkpoints for essential variables. DM completed structured digital forms. Inconsistencies prompted reviews of medical records, laboratory/imaging reports, and nursing notes. When needed, clinical consultations and AI tools (e.g., ChatGPT) supported the clarification of disease classifications and treatment data. All findings were returned to DM for validation and correction.

RESULTS:

The audit led to improved data consistency and accuracy. Though no formal correction tracking system was in place, feedback loops proved effective. Frequent issues included incorrect disease coding, incomplete treatment history, and inconsistency in timelines. A preliminary feasibility check for semi-automation through local database matching (by patient ID and exam date) was positive but not yet implemented.

CONCLUSION:

The methodology proved feasible, replicable, and effective in improving data integrity. Future steps include implementing correction logs and piloting semi-automated verification scripts. This model can be adapted to other complex domains, such as GVHD classification. A companion abstract explores the application of this methodology in that specific context.

RISK MANAGEMENT IN CELLULAR THERAPY SERVICES AS A MODEL FOR SAFETY CULTURE DEVELOPMENT

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INTRODUCTION:

Cellular therapies involve highly complex technical processes, encompassing multiple interdependent steps that are prone to failure and significant clinical risks. Strengthening patient safety practices is thus essential. Establishing a Patient Safety Pillar — grounded in the identification of high-risk activities, incident reporting, and organizational commitment to continuous improvement — is crucial to ensuring safe practices and a collaborative environment.

OBJECTIVES:

To propose an integrated Risk Management model tailored to the context of cellular therapy, focused on consolidating an institutional culture of patient safety through strategies for incident reporting and analysis, implementation of safety barriers, and fostering a just learning culture. This is a theoretical-conceptual study with a qualitative approach, based on an analysis of national and international patient safety best practices, institutional quality guidelines, and regulatory documents.

METHODS:

The model was built through five integrated steps: 1) Mapping of critical patient journey processes — from eligibility screening, cell collection and manipulation, conditioning or lymphodepletion and infusion, to outpatient follow-up — identifying vulnerabilities to adverse events; 2) Prospective risk analysis based on FMEA (Failure Mode and Effect Analysis), considering frequency, impact, and detectability, with prioritization of care and operational risks; 3) Implementation of strategies for reporting, investigating, and analyzing safety incidents, using non-punitive, just approach; 4) Alignment with safety culture pillars, including active leadership, open communication, standardization of clinical practices, multi-professional training, and valuing reporting of adverse and near-miss events; 5) Construction of a logical governance model for clinical risk, with a clear definition of responsibilities (safety team, technical coordinators, care teams), monitoring indicators, clinical audits, and longitudinal evaluation of safety outcomes.

RESULTS:

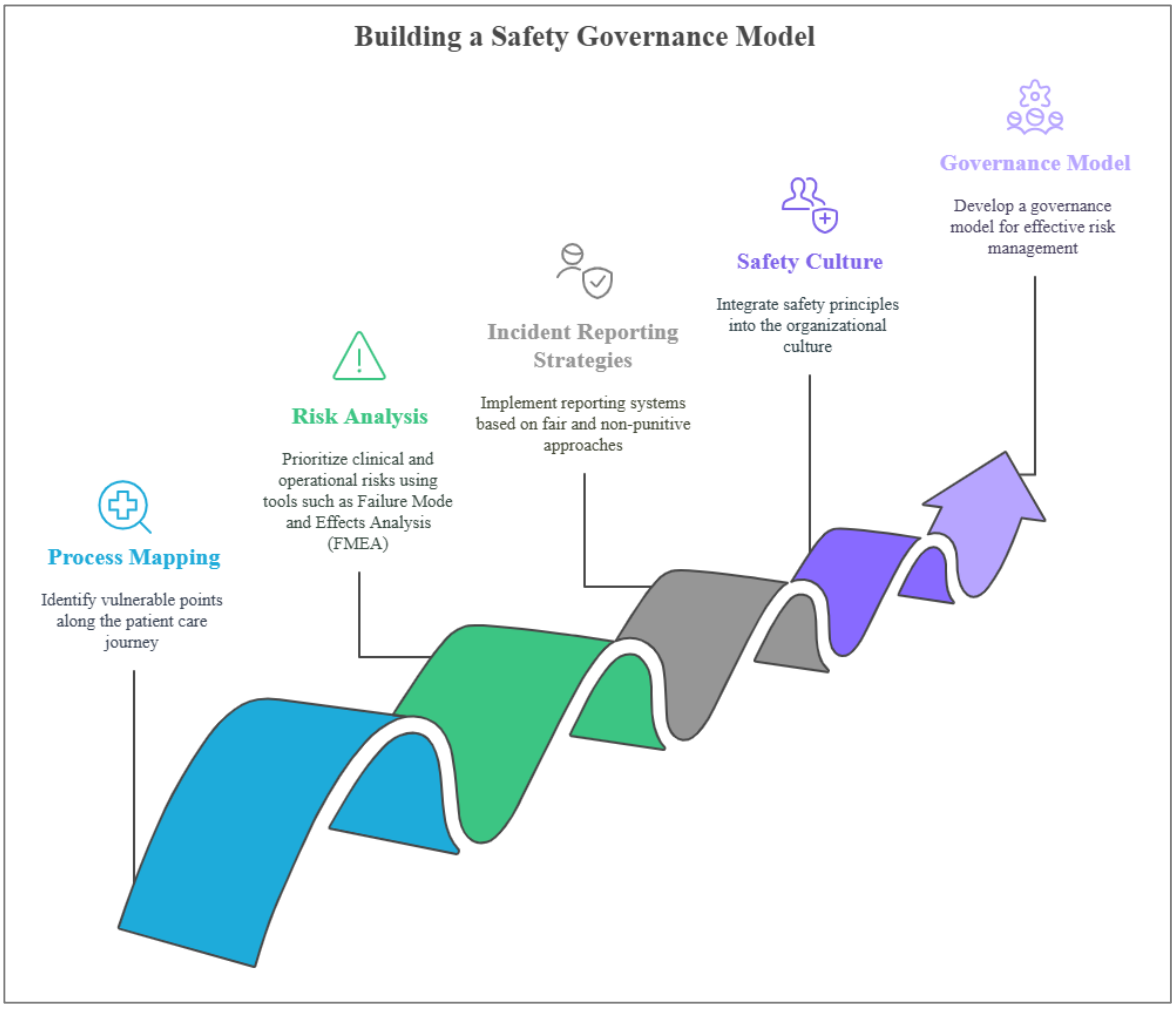
Applying this model is expected to improve the identification of vulnerabilities in critical processes, strengthen incident and near-miss reporting and analysis, standardize practices, and consolidate effective safety barriers. Leadership engagement and a just culture are also expected to enhance professional involvement, support continuous improvement, and systematize learning from failures.

CONCLUSION:

The culture of patient safety in cellular therapy and HSCT services requires a systemic, integrated, and evidence-based approach. Risk management tools like FMEA, combined with continuing education and clinical governance, allow for failure anticipation, harm mitigation, and promotion of a just culture. The proposed model may serve as a theoretical and operational guide for institutions aiming to strengthen safety in high-complexity settings, promoting safer, higher-quality, patient-centered care.

KEYWORDS: Patient Safety, Cell Therapy, Risk Assessment.

FIGURE 1


Building a Safety Governance Model
Process Mapping
Identify vulnerable points along the patient care journey
Risk Analysis
Prioritize clinical and operational risks using tools such as Failure Mode and Effects Analysis (FMEA)
Incident Reporting Strategies
Implement reporting systems based on fair and non-punitive approaches
Safety Culture
Integrate safety principles into the organizational culture
Governance Model
Develop a governance model for effective risk management

STRATEGIES TO IMPROVE DATA MANAGERS' WORKFLOW AND COMPREHENSION OF WHAT INVLOVES REPPORTING DATA TO THE INTERNATIONAL BLOOD AND MARROW TRANSPLANT RESEARCH DATA REPORTING AT A HOSPITAL IN SÃO PAULO

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INTRODUCTION:

Brazilian centers that perform hematopoietic cell transplantation (HCT) and advanced cellular therapy (ACT) have been increasingly encouraged to register data to the Center for International Blood and Marrow Transplant Research (CIBMTR), aiming to contribute to national and international research. Therefore, to achieve this goal it is essential to train data managers (DMs) and implement effective strategies to improve data submission. Objective: Present the main strategies adopted and analyze the results obtained from training DMs to improve data submission to the CIBMTR.

METHODS:

Training methods for DM's productivity, with support materials, continuous follow-ups, and standardized data submission. Training involves four progressive stages: team integration, study (course including 7 modules), observation, and practical data entry (figure 1). Digital tools such as Trello and dashboard were used for task management, with annual internal audits for quality control.

RESULTS:

From April 2024 to May 2025, 1,028 forms were completed, covering clinical data of HCT and ACT, blood bank results, and histocompatibility (table 1). Three new monitors benefited from the training program, gaining autonomy and technical skills through this educational system and supervised practice. The team was encouraged to participate in scientific events such as two Hematology and Hemotherapy Board Review's, and one International Symposium on Hemotherapy and Cellular Therapy, in addition to ten in-hospital classes for onco-hematology monitors, and monthly classes conducted by Brazilian Society of Cell Therapy and Bone Marrow Transplant (13 during this period). An efficient workflow was established for case follow-ups, focusing on form counts and average completion time. Trello was used to control DM's workflow and production, while a dashboard evaluated productivity to improve efficiency. Co-working with sectors such as Blood Bank, Lab (HLA Typing), HCT and ACT helps clarify doubts and standardize information. Annual internal audits, supported by a multidisciplinary team, identified inconsistencies, and proposed continuous improvements in data submission. Three scientific initiations were encouraged, covering CAR-T cell therapy, pre-HCT frailty scoring for elderly patients, and immunotherapy for acute lymphoblastic leukemia, with potential to become final academic projects (Thesis or Course Completion Work). Outcomes in the Continuous Process Improvement improved and accelerated the hospital's metric before the deadline requested by the CIBMTR.

CONCLUSION:

We observed improvement of technical and operational development of DM's, promoting integrity of recorded information, with the support of structured workflows, digital tools, and multidisciplinary integration. The emphasis on scientific research and technical development strengthened team engagement, resulting in a significant increase in production and potential for new studies.

KERYWORDS:

Data management; cellular therapy; data quality.

FIGURE 1. Training workflow.

TABLE 1. Number of completed forms.

FORM ID	FORMS COMPLETED	% TOTAL
2450	268	26%
2100	152	15%
2005	101	10%
2814	77	7%
2400	68	7%
2402	63	6%
2900	51	5%
2118	39	4%
4100	30	3%
2111	26	3%
4000	26	3%
2110	19	2%
4003	17	2%
4006	14	1%
2006	11	1%
2114	9	1%
2199	9	1%
2004	8	1%
2000	7	1%
2451	7	1%
2018	5	0%
2116	4	0%
2130	3	0%
4101	3	0%
2010	2	0%
2028	2	0%
2128	2	0%
2008	1	0%
2016	1	0%
2150	1	0%
2157	1	0%
4001	1	0%
TOTAL	1,028	100%

THE EFFICIENCY AMONG UNRELATED DONOR REGISTRIES – THE PERSPECTIVE OF THE BRAZILIAN BONE MARROW DONORS REGISTRY (REDOME)

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3 – Universidade Federal de Santa Maria

INTRODUCTION:

Since the creation of the first unrelated bone marrow donor registry in 1974, this activity has spread and consolidated worldwide. In 2024, bone marrow donors numbered 41.9 million, distributed among registries in 57 countries. However, the distribution of registries and hematopoietic cell transplantation (HCT) activity is heterogeneous and involves high costs, representing a great challenge for these organizations. The Brazilian Bone Marrow Donors Registry (REDOME) was established in 1993 and is the only registry of bone marrow donors in the country, maintained exclusively with public funding.

OBJECTIVE:

To examine and evaluate the efficiency of REDOME compared to other bone marrow donors registries on a global scale and to analyze its role in this context.

METHODS:

Data collected by WMDA annually for the preparation of the Global Trend Reports, referring to the activity of the year 2022, were obtained from more than 103 organizations.

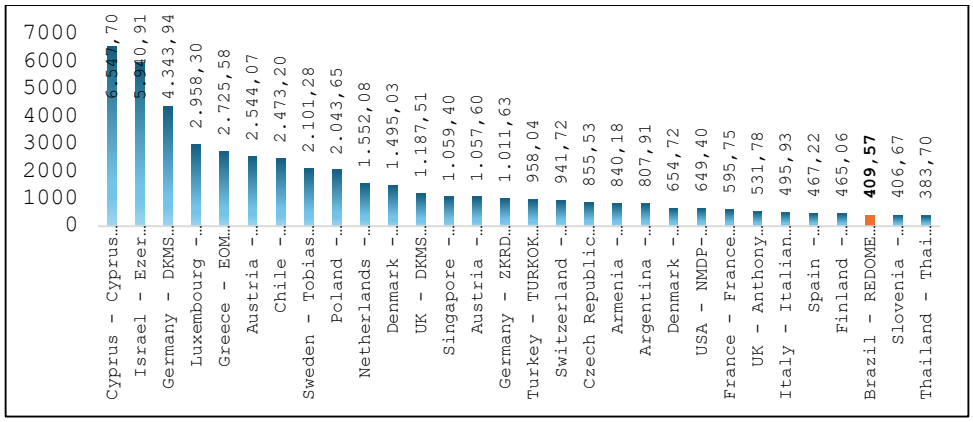
RESULTS:

The Brazilian Registry (REDOME), despite being the third largest registry of donors in the world with 5.17 million registered donors in 2022, appears as the ninth registry in number of donors per million inhabitants (23.918 donors/million inhabitants) and occupies the 28th position in number of new donors per million inhabitants (Figure 1 - 409.57 donors/million inhabitants). Analyzing some production indicators, REDOME is the 5th in the number of blood sample requests for confirmatory typing (CT). However, when considering the number of blood sample requests about the total number of donors in the registry, Brazil falls to 49th position, with 0.17 donors per 100 registered donors (Figure 2). For donor sample requests for international patients, REDOME is the 6th largest registry with 3,174 requests in 2022. In the final stages of the donation process, a total of 425 hematopoietic progenitor cell (HPC) products were obtained from REDOME (Figure 3), occupying the 10th and 18th positions for the collection of HPCs addressed to other countries, with only 77 products. In the advanced steps of the donation process, the number of cell products collected, compared to the number of samples requested for confirmatory typing, indicates that REDOME is only in position 66 among 70 analyzed registries.

CONCLUSIONS:

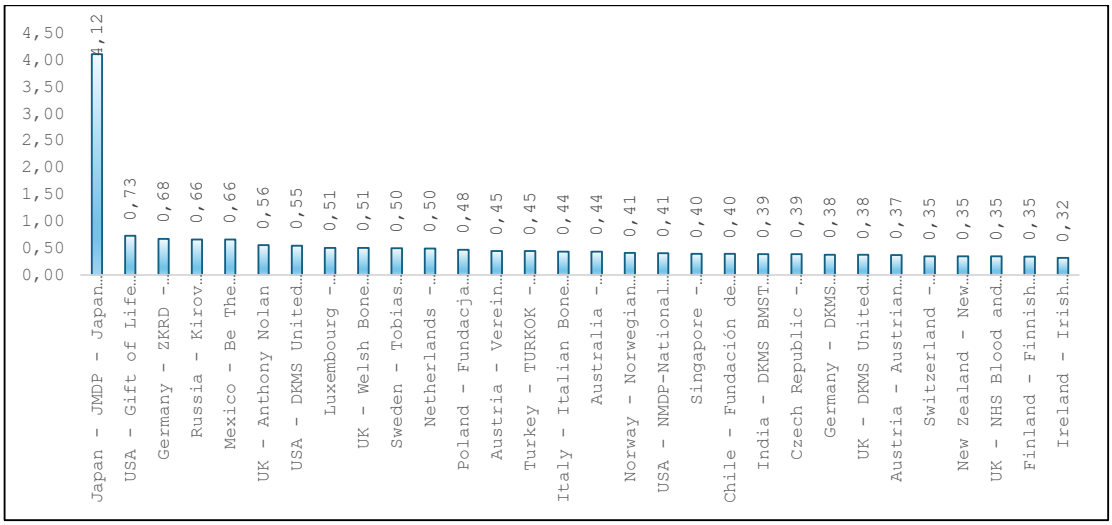
Recognized by the number of donors and genetic diversity, REDOME achieves good results in the initial steps of the registration and donation process. However, we observed a significant decline in the supply of cellular products for transplantation. These data demonstrate the challenges of analyzing efficiency in this field, which are limited by the discrepant nature of organizations and health systems, particularly in terms of registry size and activity level. Ultimately, these results could enhance the management of processes and expand the infrastructure for this activity in Brazil, thereby ensuring better care for both Brazilian and international patients.

FIGURE 1 – New donors registered in 2022 (per million inhabitants).



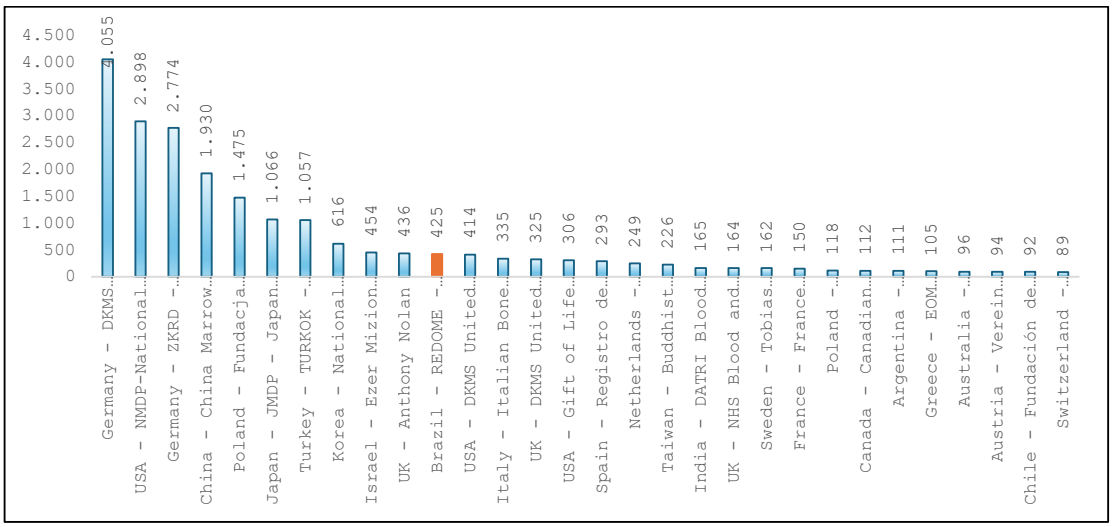
Source: WMDA – Global Trend Reports (2022) and World Data Bank (2024).

FIGURE 2 – Donor sample requests (per 100 donors).



Source: WMDA - Global Trend Reports (2022).

FIGURE 3 – Number of HPC products obtained from donors



Source: WMDA - Global Trend Reports (2022).

THE IMPORTANCE OF USING POWER BI IN MANAGING DATA ON CELLULAR PRODUCTS FOR TRANSPLANTATION WITH A FOCUS ON FINANCIAL MANAGEMENT

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INTRODUCTION

The process of providing cellular products for bone marrow transplantation involves a complex chain of operations, including donor search, exams, donor logistics organization, transportation, system maintenance, and contracts management. This requires rigorous data control for each service performed. In this context, efficient financial data management is crucial to ensure the sustainability of the Brazilian Bone Marrow Donor Registry (REDOME), optimize resources, and guarantee expenditure transparency. Power BI emerges as a strategic tool, enabling real-time data integration, facilitating decision-making, and cost monitoring.

OBJECTIVE

This study aims to show the importance of Power BI in managing financial data related to the provision of cellular products for bone marrow transplantation.

METHOD

A literature review was conducted on the application of Power BI in healthcare, with a focus on transplantation. Practical cases from institutions using the tool for financial monitoring were analyzed, including the Brazilian Ministry of Health's National Transplant System, which already employs Power BI in a simplified manner. Additionally, sample dashboards were created in Power BI, integrating the final and support cost centers of the entire operation. The data were collected from the internal management system.

RESULTS - DATA CONSOLIDATION

Automated aggregation of financial data from disparate spreadsheets, unifying cost centers for national and international patients and donors. Intuitive Visualization: Development of interactive dashboards displaying cost distribution by stage, operation type, month/year of execution, suppliers, cash flow, and key financial indicators. Identification of Inefficiencies: Detection of excessive spending, enabling contract renegotiations or new partnerships, such as the new collaboration with international donor registries and laboratories, resulted in savings of approximately $ 22 million. Predictability: Analysis of cost trends over time, where the effective monitoring of international patient operations enables the quick access of the operational efficiency from these services to reinvestment to save more Brazilian patients' lives. Transparency: Automated report generation for regulators and sponsors, improving data reliability for the institution, and enhancing accountability to stakeholders in Brazilian society.

CONCLUSIONS

Power BI proved to be an indispensable tool for financial control in project management, offering speed, accuracy, and strategic insights. Its ability to transform raw data into actionable information contributes to resource optimization, regulatory compliance, and the sustainability of healthcare services. Large-scale implementation is recommended, along with team training, to maximize its benefits. The integration of such technology not only improves operational efficiency but also has a positive impact on public transplant policies as a whole.

TRANSFORMING CLINICAL PRACTICE IN HCT AND CELLULAR THERAPY: IMPLEMENTATION OF A CARE PATHWAY MONITORING MATRIX

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The management of the care pathway in Hematopoietic Stem Cell Transplantation (HCT) and cellular therapies, such as CAR-T cell therapy, represents an increasing challenge for healthcare services in Brazil. These treatments involve high complexity, strict regulatory requirements, and the need for compliance with international quality standards, such as those established by the Foundation for the Accreditation of Cellular Therapy (FACT).

OBJECTIVE:

To reduce non-conformities and enhance the quality of the clinical processes through the implementation of a new care pathway monitoring tool for HCT and Cellular Therapy based on FACT standards.

METHOD:

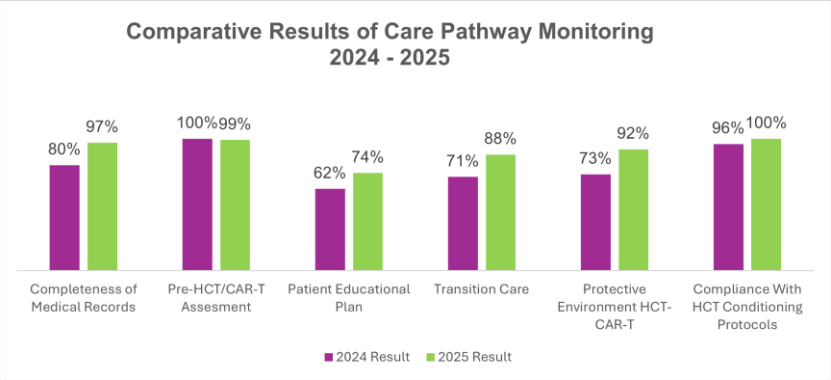
This new tool, named the Care Pathway Monitoring Matrix for HCT and Cellular Therapy was implemented in August 2024, based on a detailed mapping of all clinical and administrative processes, from the pre-transplant/CAR-T phase to post-discharge follow-up. The matrix was structured using the 5W2H methodology (What, Why, Where, When, Who, How, How much), incorporating additional components such as referenced FACT standard, institutional policy and/or protocol, monitoring responsibility, goals and outcomes, critical analysis and corrective actions. Unlike a conventional spreadsheet, which is typically limited to static data recording, the Care Pathway Monitoring Matrix was designed as an active and strategic management tool. Its structure enables continuous critical analysis, clear assignment of responsibilities, monthly goal tracking, implementation of corrective actions, and systematic evaluation of compliance with standard FACT. This approach allows near real-time monitoring of clinical processes and supports evidence-based decision-making. The monitoring of these processes included actions such as monthly training of the nursing team, verification of the protective environment, follow-up by the multidisciplinary team across different phases of care, patient education, audits, indicator management, reporting to CIBMTR, among others.

RESULTS:

The matrix was implemented in the second half of 2024 and remains in use to date. Analysis of the past 12 months of data revealed consistent improvements in monitored indicators, with notable enhancement in compliance across several critical processes. Furthermore, A comparison between the two semesters (2024/2025) demonstrated progress in the standardization of clinical practices, as illustrated in the following chart:

CONCLUSION:

The Monitoring Matrix effectively contributed to the qualification of the care pathway in HCT and CAR-T, promoting safety and adherence to international standards. As a next step, the implementation of an automated data extraction system from the electronic medical record is planned, aiming to reduce reliance on manual processes and strengthen the sustainability of the adopted strategy.



HCT and Cellular Therapy Care Pathway Matrix

Seq	Requirement	What	Flow Description	Area	Frequency	Action Responsible	Priority Level	Compliance Monitoring	Compliance Monitoring Responsible	Referenced Policy/SOP/Standard
1	Protective Environment - FACT B2									
2	Professional Training - FACT B3.1									
3	Multidisciplinary Team - FACT B4 and B7									
4	Monthly Indicator Management - FACT B4									
5	HCT Documentation - FACT B4									
6	Risk Management - FACT B4									
7	Internal Audit - FACT B4									
8	External Audit - FACT B4									
9	Third-Party Service Management - FACT B4									
10	Clinical Pharmacy Monitoring - FACT B4, B7									
11	Document Review - FACT B5									
12	Blood Bank - FACT B6, CM, C and D									
13	CAR-T Research Management - FACT B8									
14	CIBMTR Data Management - FACT B9									

January	February	March	April	May	June	July	August	September	October	November	December	Compliance Percentage	Critical Analysis	Action Plan Established

USING GVHD CLASSIFICATION CRITERIA TO ENHANCE DATA QUALITY IN HEMATOPOIETIC STEM CELL TRANSPLANTATION REGISTRIES: A STRUCTURED AUDIT APPROACH

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INTRODUCTION:

Graft-versus-host disease (GVHD) is a major complication in hematopoietic stem cell transplantation (HSCT). However, its classification in clinical registries is often inconsistent due to the complexity of diagnostic criteria and incomplete or insufficient documentation in patient charts, which hinders accurate data collection and reporting.

OBJECTIVE:

To improve the accuracy of GVHD classification through a structured data audit method, identifying common misclassifications and proposing validation strategies for diverse registry platforms.

METHODS:

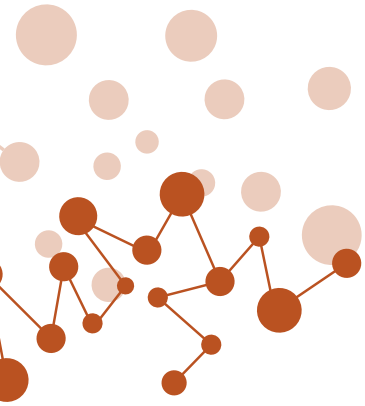
Digital audit forms were used to review GVHD-related data, focusing on organ staging in acute GVHD (aGVHD), organ involvement scoring in chronic GVHD (cGVHD), and overlap criteria. When inconsistencies arose, additional information from medical, nursing, dental, and lab records were reviewed. Clinical discussion and AI tools (e.g., ChatGPT) supported classification based on MAGIC (Mount Sinai Acute GVHD International Consortium) and NIH (National Institutes of Health) guidelines. Errors were documented and returned to data managers (DMs) for correction.

RESULTS:

The audit identified recurrent issues, including inconsistent staging in aGVHD, such as organ involvement described only as grade without adequate staging detail. Also, overlap GVHD classification errors were common - either due to simultaneous use of MAGIC and NIH criteria across organs or because chronic features were documented while acute manifestations persisted, leading to separate, inconsistent classifications. According to NIH consensus, overlap GVHD must be classified as cGVHD using NIH scoring. In cGVHD, common problems included missing or vague organ-specific scores, particularly when NIH-defined criteria required not only symptom-based assessments, but also physical examination findings and test results. These details were not consistently available in patient charts. Nursing, multidisciplinary documentation, and complementary tests were frequently consulted to identify the "worst day" of GVHD. The audit raised staff awareness, leading to an educational session on documentation standards.

CONCLUSION:

The audit process qualitatively improved GVHD data quality and increased the engagement of DMs in accurate reporting. Recurrent doubts raised to the medical team during the audit process revealed the challenges faced by DMs and highlighted the need to improve the clinical detail in patient records. By identifying frequent misclassifications and data gaps, the audit supported targeted education and fostered a culture of data accuracy. This experience highlights the value of structured audits and AI-assisted review in enhancing both data reliability and clinical documentation in HSCT settings.



HEMOTERAPY AND CELLULAR THERAPY

FIRST CASE OF LOCALIZED CRS WITH AXI-CEL: AN UNDERREPORTED CAR-T ADVERSE EVENT?

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INTRODUCTION:

Treatment for relapsed/refractory diffuse large B-cell lymphoma (R/R DLBCL) has advanced significantly, with cellular therapy—particularly CAR-T cells—representing a breakthrough, especially in early relapse. However, immune-related toxicities such as cytokine release syndrome (CRS) remain common. CRS typically presents with systemic symptoms, mainly fever, and may progress to hemodynamic instability. As experience with these therapies grows, atypical manifestations such as localized CRS have been increasingly recognized.

OBJECTIVE:

To report a case of localized CRS with cervical edema following CAR-T cell therapy and review the existing literature on this rare presentation.

METHODS:

We present a case report of a patient with R/R DLBCL who developed localized CRS post-axi-cel infusion, and a narrative literature review of similar cases.

RESULTS:

A 45-year-old woman was diagnosed with DLBCL in 2010 with cervical lymph node involvement. She received R-CHOP and radiotherapy, achieving complete remission(CR). In April 2024, she relapsed with extensive disease including hepatic lymphadenopathy, bone marrow infiltration, bone lesions, and splenic involvement. After six cycles of DHAP, she achieved a second CR. In November 2024, she presented with early relapse involving the spinal canal and abdominal nodes. Bridging therapy included ibrutinib, polatuzumab, intrathecal chemotherapy, and CNS radiotherapy. PET-CT after bridging showed complete response. In February 2025, she underwent lymphodepletion followed by axi-cel infusion. On day +5, she developed fever (CRS grade I), managed with antipyretics and tocilizumab. On day +6, dexamethasone was added due to persistent fever. She developed progressive bilateral cervical swelling with mild pain, erythema, and hoarseness, without airway compromise. CT showed enlargement of parotid and submandibular glands, pharyngeal and laryngeal thickening, and soft tissue edema. ICU admission was required for close monitoring. High-dose corticosteroids were initiated with rapid improvement. She was discharged on day +16. PET-CTs on days +30 and +90 confirmed sustained complete metabolic response.A literature review identified 26 cases of localized CRS with cervical edema, including this one—the first reported in Brazil and the first associated with axi-cel. Most cases were linked to tisagenlecleucel. Among 15 cases with available demographics, the median age was 61 years (15–75y), and 53.3% were male. Systemic CRS and fever were frequently reported. Tocilizumab was used in all cases with data, and corticosteroids in approximately 70%. Symptom resolution was observed in all patients with outcome information. Two deaths were reported, both due to disease progression.

CONCLUSION:

Localized CRS is rare but potentially severe. Cervical edema may endanger the airway and requires prompt recognition and intervention. This case emphasizes the need for awareness of atypical CRS presentations to ensure timely management.

ALTERNATIVE SOURCES OF HEMATOPOIETIC STEM CELLS: THERAPEUTIC POTENTIAL AND CHALLENGES IN EXPANSION

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INTRODUCTION:

Hematopoietic stem cells (HSCs) possess a high capacity for differentiation and are widely used to restore bone marrow function. However, the requirement for high histocompatibility between donor and recipient represents a significant barrier, limiting donor availability. To address this, alternative HSC sources such as peripheral blood and umbilical cord blood have been explored. Additionally, dendritic cells (DCs), due to their immunomodulatory role and influence on hematopoiesis, are being investigated for their potential to enhance tissue compatibility and support HSC expansion.

OBJECTIVES:

To analyze the therapeutic potential and challenges associated with the use of alternative HSC sources in patients with hematological and autoimmune diseases.

METHODOLOGY:

A literature review was conducted using the PubMed, SciELO, and Google Scholar databases, employing the descriptors "Hematopoietic Stem Cells," "Hematopoietic Stem Cell Transplantation," and "Stem Cell Sources," according to DeCS and MeSH. Articles published between 2020 and 2025 in Portuguese and English were considered. Of the 10 initially selected articles, 3 were excluded for not aligning with the proposed theme, resulting in a final sample of 7 articles analyzed.

RESULTS:

HSCs are essential in the treatment of various hematological and autoimmune disorders. While traditionally sourced from bone marrow, this method requires high HLA compatibility, which limits donor availability and increases the risk of graft-versus-host disease (GVHD). Alternative sources such as peripheral and umbilical cord blood are more accessible and less invasive but present challenges such as lower cell counts and variable clinical efficacy.

Dendritic cells have emerged as a promising tool to support HSC acquisition and expansion due to their ability to produce cytokines (e.g., thrombopoietin, IL-6, IL-12) and mediate hematopoietic interactions via adhesion molecules. When these mechanisms are replicated ex vivo, they allow for experimental control, potentially improving safety and therapeutic outcomes. Nonetheless, clinical application remains limited due to complex production protocols, challenges in ensuring quality and viability of expanded cells, inadequate artificial bone marrow matrices, scarcity of clinical trials, and incomplete understanding of the molecular pathways involved in DC-HSC interactions.

CONCLUSION:

Alternative HSC sources such as peripheral and umbilical cord blood offer promising therapeutic potential but are constrained by technical and biological limitations. The use of dendritic cells to support HSC expansion is an innovative approach with encouraging experimental results; however, further research is needed to elucidate underlying mechanisms and establish standardized protocols. Advances in ex vivo expansion techniques and well-designed clinical trials are crucial to reducing the dependence on HLA compatibility and broadening access to hematological therapies.

KEYWORDS: Hematopoietic Stem Cells; Stem Cells Alternative Sources, HLA compatibility

AUTOMATIC MONITORING OF TEMPERATURE AND HUMIDITY IN A CELL PROCESSING CENTER

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INTRODUCTION:

The Cell Processing Center must ensure that adequate temperature and humidity are maintained. Automatic monitoring of temperature and humidity in facilities is an increasingly common practice to ensure quality, traceability and compliance with regulatory standards, especially when these parameters directly influence the quality of the material during its processing and storage. As opposed to manual recording, automated recording allows continuous monitoring, graphic and interactive visualization in real time and remote access.

OBJECTIVE:

to implement automatic and continuous recording of room temperatures, refrigeration chambers, -80°C freezers, and ambient air relative humidity using the Elipse E3 Viewer® software (Elipse®) and additionally, to perform periodic assessments to verify the agreement between the sensors of the automated system and the sensors of the equipment or environments, using a certified quality equipment.

METHODOLOGY:

A qualification plan was developed establishing the design, performance measures and expected results. The temperature and ambient air relative humidity data of the sensors used as standard and of the sensors used by the Elipse® were recorded at the same time for comparison. The parameters evaluated were the average differences in temperature and ambient air relative humidity between the two sensors. The average temperature differences should not exceed ± 3°C for room temperatures, ± 2°C for refrigeration chambers and ± 5°C for -80ºC freezers. The average differences in ambient air relative humidity should be no more than ± 8%. These goals were used both for the qualification of the sensors and for the periodic evaluation of their operation, carried out monthly at all points monitored by the Elipse®.

RESULTS:

All the monitoring points outlined by regulatory standards were implemented, including two for room temperatures, two for ambient air relative humidity, two for refrigeration chambers and five for -80°C freezers. The results found in the implementation of automatic recording were in accordance with the qualification plan definitions. In addition to the possibility of remote access by the staff, remote monitoring is performed permanently, in real time, on a screen located in the institution's engineering office. Periodic monitoring of comparison between standard sensors and Elipse® sensors is being performed once a month and has proven effective in demonstrating agreement between the sensors. Occasionally, in the event of discrepancies between the measurements, interventions were carried out to check the placement of the sensors and/or detect malfunctions.

CONCLUSION:

Continuous monitoring of temperatures and humidity was successfully implemented in this cell processing center. This monitoring allows for the real-time detection of temperature and humidity variation, contributing to the safety and maintenance of the quality of the cell therapy products processed and stored at this facility.

AXICABTAGENE CILOLEUCEL IN SECONDARY CENTRAL NERVOUS SYSTEM DIFFUSE LARGE B CELL LYMPHOMA: A CASE REPORT

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INTRODUCTION:

Diffuse Large B-Cell Lymphoma (DLBCL) is the most common subtype of non-Hodgkin lymphoma. Secondary CNS involvement in patients with DLBCL is rare, with an estimated incidence of approximately 5% and it often happens in the first disease relapse (87%). The prognosis of SCNSL is very poor and nowadays there are still very few effective treatment options for these patients. Moreover, clinical trials usually exclude patients with CNS involvement. Hence, new treatment strategies for SCNSL remain an unmet clinical need.

OBJECTIVE:

To describe the case of a patient with a non germinal center DLBCL, relapsed in the CNS, successfully treated with a chimeric antigen receptor (CAR) T-cell therapy, Axicabtagene ciloleucel (Axi-cel).

CASE DESCRIPTION:

Male patient, 57 years old, was diagnosed with a non germinal center DLBCL with a bulky mass in his right inguinal region in April/2020. He was treated with 6 cycles of R-CHOP followed by radiotherapy in a residual lesion in the inguinal region reaching a complete metabolic response (CMR) by the end of the treatment. In July/2021, the patient developed neurological symptoms (paresis of his right arm and leg, and aphasia) and new lymph nodes enlargement, thus being diagnosed with CNS and systemic DLBCL relapse. The patient received 3 cycles of high-dose methotrexate (HD-MTX), cytarabine, rituximab achieving a CMR. The patient was then referred to our center and submitted to an autologous stem cell transplantation (ASCT) in October/2022 as consolidation therapy with carmustine and thiotepa as conditioning regimen. By day 33 and 4 months after ASCT, MRI and PET-CT evaluations demonstrated complete remission of disease. In September/2023, the patient presented new neurological symptoms and a MRI showed a new lesion in the brain that came up proven as a new SCNSL relapse after a stereotaxic cerebral biopsy. A PET-CT showed systemic relapse as well. The patient was treated with 5 cycles of HD-MTX, cytarabine, rituximab combined with ibrutinib. After the fifth cycle in january/2024, the patient achieved a CMR and maintained treatment with ibrutinib monotherapy. We then proceeded to CAR-T Cell therapy as consolidation therapy, using Axi-cel. In december/2024, the axi-cel product was infused (2x108 CD3/kg). The patient presented with grade 1 CRS, successfully managed during hospitalization and was discharged by the 10th day after axi-cel infusion. MRI and PET-CT evaluation by 30 days and 6 months after CAR T-cell showed sustained CMR. The patient remains in remission, with no signs of disease progression 6 months after the infusion of axi-cel. Conclusion: Relapsed DLBCL with secondary CNS involvement has very poor prognosis with high rates of relapse even after ASCT. Axi-cel is a new treatment strategy that has shown high efficacy in the treatment of relapsed DLBCL and might be a good option in those cases with CNS involvement as well.

KEYWORDS: CAR-T cell; DLBCL; CNS involvement.

CHIMERIC ANTIGEN RECEPTOR T CELL THERAPY IN A BRAZILIAN CANCER CENTER: REAL WORLD PATIENTS CHARACTERISTICS

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INTRODUCTION:

Chimeric antigen receptor T (CAR-T) cell therapies are an emerging treatment option for patients with refractory and relapsed hematological malignancies, such as diffuse large B-cell lymphoma (DLBCL), follicular lymphoma (FL), B-cell acute lymphoblastic leukemia (B-ALL), and multiple myeloma (MM). Although approved by Brazilian health Regulatory Agency (ANVISA) since 2022, clinical experience with CAR-T in Brazil is still limited.

OBJECTIVES:

Our goal is to share our clinical experience with commercial CAR-T therapy at a Cancer Center in Sao Paulo, Brazil, and offer comprehensive insights into treatment timing, disease status, and resource allocation for this therapy, encouraging discussion to enhance cost-effectiveness and broader access for patients.

METHODS:

We retrospectively analyzed electronic records from 24 patients treated with CAR-T therapy at ACCamargo Cancer Center between April 2023 and December 2024.

RESULTS:

Among the 24 patients analyzed, the median age at the time of CAR-T infusion was 50years (18-77 years). Most patients were male (63%). Patients received at least 3 prior lines of therapy, with a median of 4 lines (ranging from 3-7). Eleven patients received prior hematopoietic stem cell transplantation (HSCT), being 9 allogeneic and 2 autologous. Diffuse Large B-Cell Lymphoma accounted for 75% of the cases, with Tisagenlecleucel (tisa-cel) being the most widely used cell therapy product (58%). Eighty-eight of patients needed bridging therapy. The median brain-to-vein time of 137 days (67-357 days). The average hospitalization time was 14 days. Regarding immediate complications, 91.6% of patients had Cytokine Release Syndrome (CRS), with the majority (54.1%) presenting CRS grade 02. In total, 83.3% required therapy with Tocilizumab. In addition, 15 patients (62.5%) presented Immune Effector Cell-Associated Neurotoxicity Syndrome (ICANS), with one death related to convulsive status in a patient receiving AxiCel.

CONCLUSION:

Our findings demonstrated an acceptable safety profile of CAR-T Cell therapy in this population. However, there was a prolonged brain-to-vein time, reinforcing the importance of early referral and coordinated efforts across institutions and health care providers to ensure broader and timely access to this therapy, improve clinical outcomes, and maximize cost-effectiveness in real-world settings.

KEYWORDS: CAR-T cell therapy, Lymphoma, Multiple Myeloma, B-cell acute lymphoblastic leukemia.

TABLE 1. CAR T cell Patients characteristics

Total number of patients	24
Age (years). Median (range)	50 (18-77)
Sex, n (%)	
Female	9 (38)
Male	15 (63)
Disease, n(%)	
Diffuse Large B-cell Lymphoma	18 (75)
Acute lymphoblastic leukemia	3 (13)
Multiple Myeloma	1(4)
Follicular lymphoma	2(8)
Prior auto-HSCT, n (%)	9 (38)
Prior alo-HSCT, n (%)	2 (8)
CAR T-cell indication	
2nd line	0 (0)
≥3rd line	24 (100)
Nº of prior lines of therapy,median(range)	4 (3-7)
CAR T-cell product, n(%)	
Tisa-cel	14 (58)
Axi-cel	9 (38)
Cilta-cel	1 (4)
LDH pre-CART-cell (IU/L)	230 (184-874)
Bridging chemotherapy, n(%)	
Yes	21(88)
No	3 (13)
Brain-to-vein time (days), median (range)	137 (67-357)
Post-CAR T-cell outcomes	
CRS grade, n(%)	
0	2 (8)
1	5 (21)
2	13 (54)
3	3 (13)
ICANS grande,n (%)	
Yes	15 (63)
No	9 (38)
Use of tocilizumab, n(%)	
Yes	20 (83)
No	4 (17)
Use of dexamethasone, n(%)	
Yes	17 (71)
No	7 (29)

Abbreviations: Axi-cel,axicabtagene ciloleucel; HSCT,haematopoietic stem cell transplantation;CRS, cytokine release syndrome; ICANS,effector cell-associated neurotoxicity syndrome;LDH,lactate dehydrogenase;Cilta-cel, ciltacabtagene autoleucel; Tisa-cel, tisagenlecleucel.

EXPERIENCE REPORT OF THE FIRST CAR-T IN A CELLULAR PROCESSING CENTER IN THE NORTHEAST

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1. Centro de Hematologia e Hemoterapia do estado do Ceará

INTRODUCTION:

Immunotherapy using T cells modified with a chimeric antigen receptor (CAR-T) has gained prominence in recent years for the treatment of onco-hematological neoplasms, as it offers curative potential for patients with relapsed or refractory disease. Through genetic engineering, T cells are reprogrammed to identify and destroy cancer cells that express specific targets such as CD19 or the B-cell maturation antigen (BCMA). Unfortunately, access to this treatment remains limited to a small number of patients worldwide. The main factors contributing to this limited access include the high cost and the slow incorporation of innovative therapies into various healthcare systems. The operational management of this therapy is a complex process involving multiple stages and multidisciplinary teams to ensure that treatment is safe, effective, and compliant with regulatory standards.

AIM: To report the experience of administering CAR-T manufactured by the pharmaceutical industry from the perspective of a Cell Processing Center (CPC) located in the Northeast region of Brazil.

METHODOLOGY:

A reflective description focused on the contextualization of a professional experience.

RESULTS:

In order to obtain certification from pharmaceutical companies and become a reference center for the use of this therapy, the apheresis unit, the CPC, and the Transplant Center (TC) underwent a rigorous process of audits, documentation adjustments, technical team training, and equipment acquisition to meet industry requirements. The first certification was granted in April 2024, and the first delivery occurred in January 2025. Through a specific online portal, we monitored the entire process from medical prescription to real-time GPS-tracked transportation. The product arrived via São Paulo but required clearance by the local finance department, which delayed its arrival. It was transported in a dry shipper, and all procedures for identity verification, quality control, and transfer to a vapor-phase nitrogen freezer at -150 °C, as outlined in the pharmaceutical manual and internal technical guidelines, were completed and reported on the portal. The existing workflow for requesting cell therapy products was maintained. The infusion took place on February 1st, 2025, at the partner TC in a patient with Acute Lymphoblastic Leukemia. The CPC team prepared the product, in a dry shipper validated by our service. Transport and delivery to the TC occurred without incident. Temperature monitoring of the dry shipper, water bath, and environment was strictly performed according to protocol. Bedside thawing by the CPC nurse and infusion by the TC team were successful. The patient was later discharged and remains under follow-up.

CONCLUSION:

In this pioneering experience in our state, we combined study, commitment, and dedication to deliver high-quality work, contributing to the implementation of CAR-T therapy, an important milestone in healthcare in the Northeast.

KEYWORDS: Cell therapy; Chimeric Antigen Receptor; T lymphocytes.

IMPACT OF SHORT-TERM TRANSFER FROM NITROGEN TO -80°C FREEZER ON THE VIABILITY OF CRYOPRESERVED CELLULAR THERAPY PRODUCTS: AN IN VITRO STUDY

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INTRODUCTION:

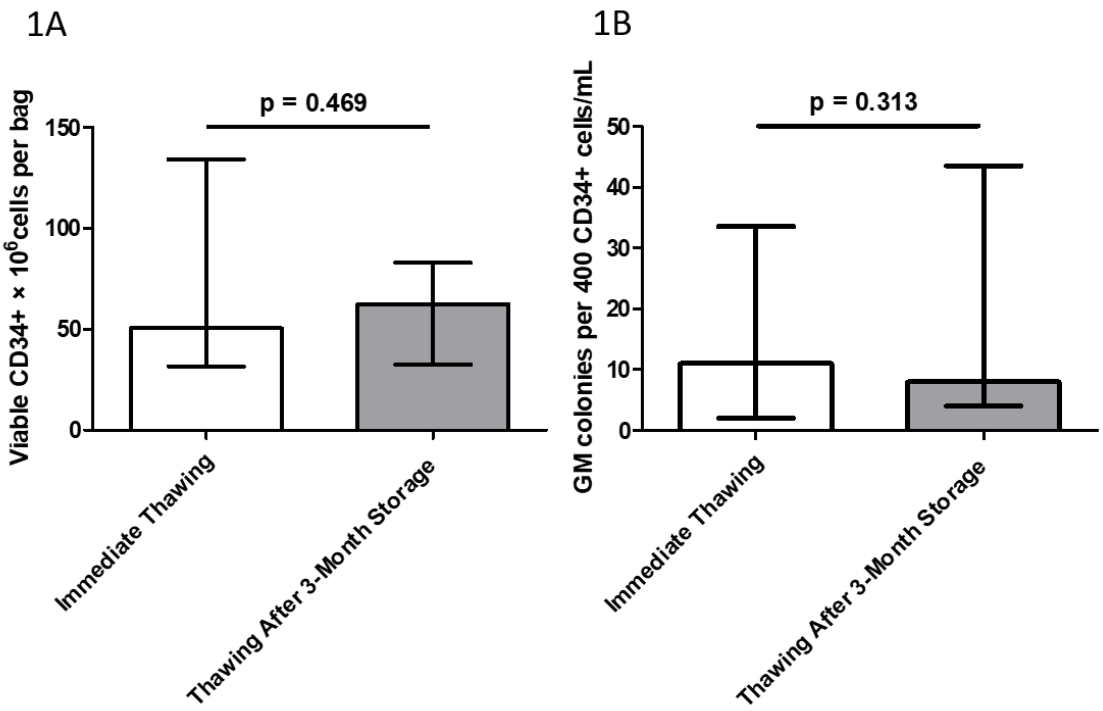
Liquid or vapor nitrogen is the gold standard for the storage of cellular therapy products, ensuring long-term viability and stability. However, contingency planning remains a challenge, as each cryopreserved unit requires a corresponding empty slot in other tank to ensure redundancy in case of failure. Aim: This study aimed to assess the impact of short-term transfer of cryopreserved cell therapy units from nitrogen to a -80°C freezer, using in vitro tests.

METHODS:

This was an experimental study that included cellular therapy units from individuals who had been referred for autologous stem cell transplantation. Cryopreservation had been conducted at a single processing facility between 2014 and 2024. Cryopreserved units with authorized discard, aliquoted in at least two bags, were included. One bag was thawed immediately after removal from vapor nitrogen tank, while the paired bag was stored at -80°C for three months before being thawed. The -80°C freezer was continuously monitored; the door had been opened a few times for brief periods, but temperatures had never exceeded -76°C. Thawing was carried out using a 37°C water-bath. The bag was placed on ice, and a resuspension solution at double the final concentration (3% human albumin, 2% hydroxyethyl starch 130/0.4, and 2.5% ACD) was slowly added in a 1:1 volume ratio. Samples were collected for total nucleated cell count, CD34+ immunophenotyping, and colony-forming assay. Data were described using medians with interquartile ranges or percentages, and continuous variables were compared using the non-parametric Wilcoxon paired test. Results: To date, seven bag pairs have been analyzed. The median total nucleated cell count was 292.9 (272.4) x 108 in the liquid nitrogen group and 270.4 (255.9) x 108 in the -80°C group (p = 0.109). Viable CD34+ cell counts were comparable between groups, with medians of 50.4 (102.5) x 106 and 62.5 (50.5) x 106, respectively (p = 0.469; Figure 1A). The number of GM colonies did not significantly differ, with 11 (31.5) colonies per 400 CD34+ cells/mL in the nitrogen group and 8 (39.5) colonies per 400 CD34+ cells/mL in the -80°C group (p = 0.313; Figure 1B). Conclusion: These preliminary results suggest that -80°C freezers may serve as a short-term contingency storage strategy for cell therapy products, if maintained under strict conditions to prevent transient warming events. Nevertheless, the limited number of thawed units and lack of in vivo validation underscore the need for further evidence before clinical guideline implementation.

KEYWORDS: Cryopreservation; Cell therapy; Storage contingency

FIGURE 1. Comparison of post-thaw viable CD34+ cell recovery (A) and clonogenic potential (B) between cryopreserved units thawed immediately after removal from vapor-phase liquid nitrogen storage and those stored at -80°C for 3 months prior to thawing



IMPLEMENTATION OF A CHECKLIST FOR HEMATOPOIETIC STEM CELL COLLECTION: STRATEGIES FOR PROCEDURE SAFETY

Wlly Clecia da Cruz Santos[1], Fabiana Nogueira de Oliveira[2]

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INTRODUCTION:

The implementation of a checklist as a strategy to increase safety in the collection of hematopoietic stem cells (HSC), a complex and high-risk procedure. Standardizing the steps and ensuring integrated work among multidisciplinary teams are essential to prevent failures that could compromise the success of the transplant and patient safety. Inspired by the World Health Organization's (WHO) surgical safety checklist, this study proposes a tool that organizes actions, strengthens communication, and reduces risks.

OBJECTIVE:

To develop, validate, and implement a specific checklist for HSC collection to ensure greater patient safety during the procedure.

METHODOLOGY:

A psychometric study aimed at collecting evidence for content validity and response process. It was conducted in an internationally accredited hospital institution with a Hematopoietic Stem Cell Transplantation (HSCT) service. The construction of the checklist was based on: bibliographic research in databases such as PubMed, Scielo, and LILACS; direct observation of collection procedures; discussions with team professionals; content validation by hematology experts; and the application of the checklist in fifteen autologous collection procedures.

RESULTS:

The instrument was structured in three stages: Sign in (before starting the collection), Time out (pause before the invasive procedure), and Sign out (before the procedure ends). Initially with 15 items, the checklist was expanded to 19 after validation, with all items having a content validity ratio (CVR) above 0.80. Items showing duplication were excluded, and new ones focusing on collection safety were added, such as: patient identification, signed consent form, equipment functionality, vital signs verification, allergy check, confirmation of CD34+ result, team preparation, and material conditions. After one year of implementation, the results indicated increased patient safety, process standardization, improved communication, and better traceability of the procedure. Despite initial resistance from some team members, continuous use demonstrated the benefits of the tool, such as clearer responsibilities and fewer errors. The systematization promoted team integration and strengthened the safety culture.

CONCLUSION:

The checklist significantly contributed to improving the quality of care in HSC collection, promoting safer and more effective practices. The active involvement of professionals in its development was crucial for its acceptance and effectiveness in daily clinical practice. This ensured both safety and quality in patient care, as well as empowerment in safety practices to provide secure assistance during Hematopoietic Stem Cell Transplantation (HSCT).

KEYWORDS: Hematopoietic Stem Cell Collection, Patient Safety, Checklist Implementation

LOCALIZED CYTOKINE RELEASE SYNDROME (L-CRS) FOLLOWING CART CELL THERAPY IN A PATIENT WITH PRIMARY CENTRAL NERVOUS SYSTEM LYMPHOMA: A CASE REPORT

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ABSTRACT

Chimeric antigen receptor T-cell (CAR-T) therapy has transformed the treatment landscape for refractory hematologic malignancies. Despite its success, CAR-T therapy can result in significant adverse events, including cytokine release syndrome (CRS) and immune effector cell-associated neurotoxicity syndrome (ICANS). We report a rare case of localized CRS (L-CRS) in a patient with primary central nervous system lymphoma (PCNSL) treated with axicabtagene ciloleucel. The patient developed cervical edema without underlying tumor involvement, suggesting potential alternative pathogenic mechanisms, including off-target CAR-T cell effects. Prompt corticosteroid therapy led to symptom resolution and avoided systemic complications. This report underscores the need for early recognition and individualized management strategies for L-CRS.

INTRODUCTION

CAR-T cell therapy has revolutionized the treatment of refractory hematologic malignancies, providing durable responses in patients who previously lacked effective therapeutic options. However, adverse events such as Cytokine Release Syndrome (CRS) and Immune Effector Cell-Associated Neurotoxicity Syndrome (ICANS) remain significant clinical challenges. This report describes a case of localized CRS (L-CRS) - a rare and poorly understood manifestation - in a patient who received axicabtagene ciloleucel (Yescarta®) for the treatment of primary central nervous system lymphoma (PCNSL).

CASE REPORT

A 58-year-old female was diagnosed with PCNSL in 2020 and initially treated with high-dose chemotherapy using a modified MATRix protocol, followed by autologous hematopoietic stem cell transplantation with a thiotepa-based conditioning regimen, achieving complete remission. She relapsed in May 2023 and received a second-line regimen of four cycles of rituximab, methotrexate, and cytarabine, which failed to induce remission. Disease progression continued despite third-line therapy with rituximab, ifosfamide, and etoposide (two cycles). Subsequently, the patient underwent whole-brain radiotherapy (34 Gy), which was interrupted prior to the final fraction but achieved a complete response.

Given her disease course and lack of durable response to prior lines of therapy, the patient was referred for CAR-T cell therapy as a salvage and consolidation strategy. She received axicabtagene ciloleucel (Yescarta®) on 27/03/2024.

On day 1 post-infusion (D+1), she developed grade 1 CRS, presenting with isolated fever. By D+2, the CRS progressed to grade 2 with persistent fever and mild hypoxia, managed with a single dose of tocilizumab. On the morning of D+4, she developed cervical edema that progressed rapidly over a few hours (Figure 1A-B), accompanied by throat discomfort and mild respiratory distress, though without oxygenation impairment.

Imaging confirmed subcutaneous edema and soft tissue thickening in the cranio-cervical region.

Ultrasonography revealed skin thickening and diffuse increased echogenicity of the subcutaneous tissues, with fluid layers extending into the cervical muscle planes, consistent with edema. Computed tomography (CT) showed thickening and densification of soft tissues in the perimandibular and mentonian regions, thickening of the superficial cervical fascia and platysma bilaterally, and densification of deep adipose planes involving multiple cervical and pharyngeal compartments (Figure 1C-D). No evidence of infection was found on serological tests.

She was treated with dexamethasone 10 mg every 8 hours, with progressive improvement and resolution of the edema within 72 hours (Figure 2). On D+5, she developed grade 3 ICANS (ICE score = 0), prompting intensification of immunosuppression with dexamethasone 10 mg every 6 hours. After stabilization, steroids were tapered and discontinued by D+13 following resolution of neurotoxicity. The patient was discharged in remission and continues under outpatient follow-up.

DISCUSSION

Localized CRS is a rare and poorly described adverse event, often linked to focal inflammation induced by CAR-T cell activation within tumor microenvironments. However, in this case, there was no lymphomatous involvement in the cervical region, suggesting alternative mechanisms in L-CRS pathogenesis. Redistribution of CAR-T cells may lead to on-target, off-tumor toxicity, where healthy tissues expressing the target antigen are inadvertently affected.

This case exemplifies L-CRS with acute cervical edema, rapid progression, and involvement of deep myoadipose planes, features supported by imaging. Similar findings have been reported in the absence of local tumor burden [1], supporting the hypothesis that mechanisms beyond microenvironment activation contribute to L-CRS. Off-target effects and aberrant immune activation may be key contributors.

While interleukin-6 blockade with tocilizumab is often effective in systemic CRS, its efficacy in L-CRS is inconsistent. Corticosteroids have emerged as the primary treatment, particularly in cases where airway compromise is a concern [2–3]. In this case, early initiation of dexamethasone led to rapid clinical improvement and likely prevented airway obstruction, a recognized risk of L-CRS.

The absence of predictive biomarkers for L-CRS complicates early diagnosis and management. Most available biomarkers pertain to systemic CRS, underscoring the need for improved diagnostic tools. Early clinical recognition, combined with prompt imaging, is essential. Severe presentations requiring airway protection, including intubation, have been reported, highlighting the importance of proactive management.

This case underscores the need for greater awareness of L-CRS as a potentially serious complication. Clinical vigilance, early corticosteroid therapy, and multidisciplinary management are crucial to avoid escalation. Further research and case documentation are needed to clarify L-CRS pathophysiology, identify risk factors, and establish evidence-based management strategies.

CONCLUSION

Localized CRS is a rare but potentially severe toxicity associated with CAR-T cell therapy. Prompt recognition and management, particularly with corticosteroids, are vital to prevent complications and preserve therapeutic benefit. Additional studies are needed to elucidate the mechanisms behind L-CRS and to identify predictive markers that could improve early diagnosis and guide therapy.

CONFLICTS OF INTEREST AND FUNDING: The authors declare no conflicts of interest. No funding was received for the preparation of this manuscript.

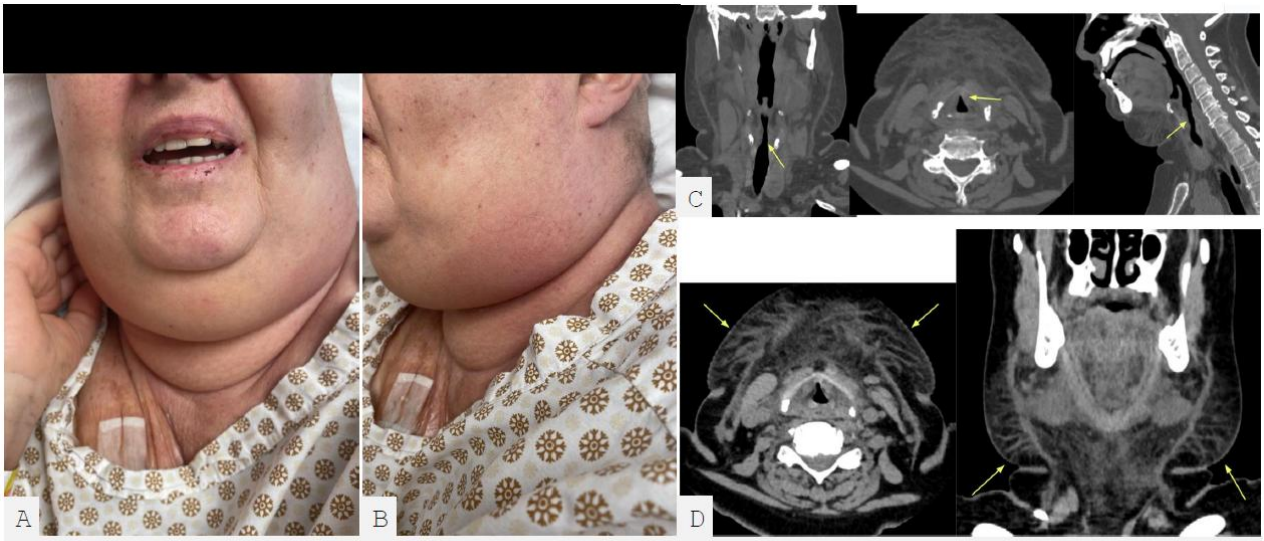

Fig.1

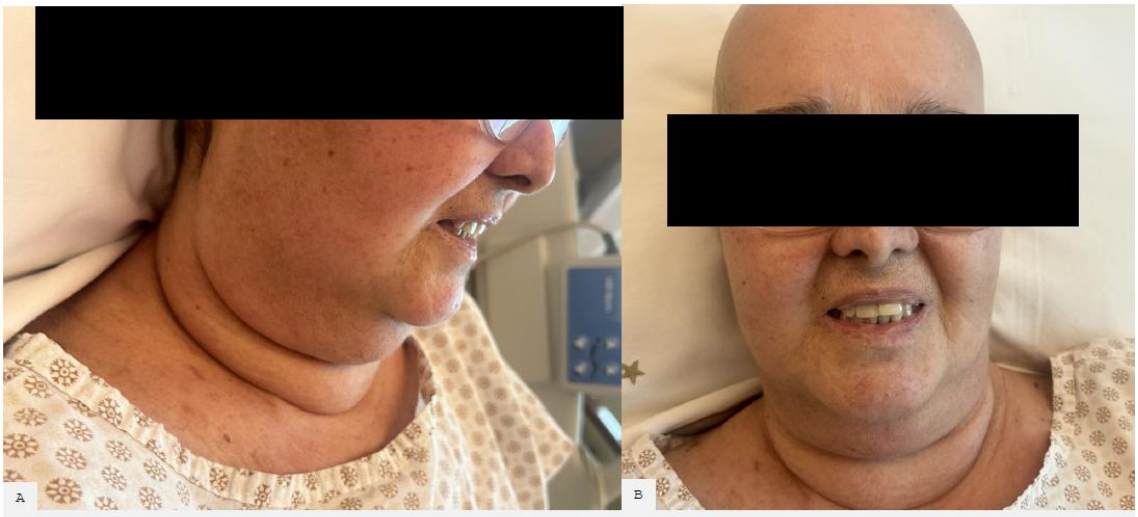

Fig.2

MEDICAL ACTION IN THE IMPLEMENTATION OF HEMATOPOIETIC STEM CELL COLLECTION BY APHERESIS IN A 100% SUS TRANSPLANT CENTER: EXPERIENCE REPORT

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is indicated for several diseases and significantly impacts patient prognosis. Hematopoietic progenitor cells (HPC) can be obtained by bone marrow aspiration, peripheral blood (apheresis), or umbilical cord, with transplantation classified as autologous, allogeneic, or syngeneic. In Minas Gerais, apheresis collection is often performed by outsourced blood centers, which can limit access and prolong waiting times. The implementation of collection by the SUS Transplant Centers themselves emerges as an alternative to optimize the process, reduce hospitalizations, and minimize associated risks.

OBJECTIVE:

To report the experience of transplant physicians in supporting and conducting the implementation of HPC collection by apheresis at a 100% SUS Transplant Center in Minas Gerais.

METHOD:

Report on the medical team's experience in implementing HPC collection by apheresis at a 100% SUS Transplant Center.

RESULTS:

Implementation began in June 2023, with transplant physicians and hemotherapists guiding the nursing team and the blood bank/transfusion agency. The main interface was with the Transfusion Agency and the Cell Processing Laboratory, ensuring the quality and safety of the procedure. The Standard Operating Protocol defined the physician responsible for collection, who performed the clinical evaluation of the donor, defined the chemomobilization regimen, monitored the collection, and managed complications. Medical supervision ensured the suitability of the material for immediate infusion (fresh), integrating with the cell processing and transplantation team. This action enabled quick and effective decisions, essential for the success of the procedure and the viability of the graft. Coordination with the pre-BMT physician optimized the evaluation and referral of patients. In October 2024, the first hospitalization with collection and infusion during the same admission took place, reducing the length of stay, invasive procedures (such as the implantation of two central venous accesses), and travel. By May 2025, seven successful collections had been performed, with confirmed neutrophil engraftment, and all patients were discharged from the hospital, demonstrating the effectiveness of the implementation. This initiative reflects the Center's continuous search for improvement and incorporation of new procedures, expanding its operational capacity and generating opportunities to broaden service delivery to the population.

CONCLUSION:

Medical involvement in HPC collection by apheresis is essential to ensure donor safety, product quality, and transplant success. Clinical evaluation, supervision of the procedure, and partnership with the multidisciplinary team ensure efficiency and humanization. This experience reinforces the importance of medical support in optimizing care at the 100% SUS Transplant Center, promoting sustainability and qualification of services.

KEYWORDS:

Hematopoietic Stem Cell Transplantation; Apheresis; Medical Practic.

MICROBIOLOGICAL MONITORING OF LEUKAPHERESIS PRODUCTS FOR HEMATOPOIETIC STEM CELL TRANSPLANTATION AND CELL THERAPIES: A RETROSPECTIVE EVALUATION (2023–2024) AT A FACT-JACIE ACCREDITED CENTER

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BACKGROUND:

In the context of advanced therapies and hematopoietic stem cell transplantation, the microbiological safety of cellular products is a critical requirement for their release and clinical application. The occurrence of microbiological contamination poses a direct risk to patient safety, potentially compromising cell viability, diminishing therapeutic efficacy, and significantly increasing the likelihood of severe adverse events such as systemic infections. Within this framework, the maintenance of aseptic conditions throughout all stages of the production chain is essential to ensure product integrity and therapeutic success.

OBJECTIVE:

This study aims to assess the absence of microbiological contamination in leukapheresis-derived cellular products, considering the stages of collection, packaging, processing, and cryopreservation, in accordance with ANVISA guidelines and international FACT-JACIE standards.

METHODS:

Between January 2023 and December 2024, a total of 264 apheresis bags were processed for cryopreservation in the context of cellular therapies, including CAR-T cell therapy and hematopoietic stem cell transplantation. In 2023, 158 apheresis bags were received, resulting in the cryopreservation of 614 aliquots (46 allogeneic and 112 autologous), with 414 aliquots released for infusion. Among these, 101 bags contained autologous peripheral blood hematopoietic progenitor cells (PB-HPCs) (63.9%), 46 contained related allogeneic PB-HPCs (29.1%), and 11 contained lymphocytes (7.0%). Additionally, 17 fresh infusions were performed. In 2024, 106 bags were processed, yielding 391 cryopreserved aliquots (284 allogeneic and 107 autologous), of which 258 were infused. These included 76 autologous PB-HPCs, 21 related allogeneic PB-HPCs, 2 unrelated allogeneic PB-HPCs, and 7 lymphocyte products. Eleven additional collections were used for fresh infusion during the same period. All processed bags underwent microbiological quality control testing using the BACTEC™ system (BD), with specific culture vials designed to detect aerobic and anaerobic bacteria, fungi, and yeasts.

RESULTS:

No cases of microbiological contamination were identified in any of the collected and cryopreserved bags during the analyzed period.

CONCLUSION:

The findings demonstrate the robustness of institutional Good Manufacturing Practice protocols across all stages of processing, reinforcing the reliability of microbiological quality control procedures and contributing directly to the safety and efficacy of cell-based therapies.

KEYWORDS: Cell therapy, sterility testing, leukapheresis

MYELODYSPLASTIC SYNDROME AFTER CAR T-CELL THERAPY: RELATE OF TWO CASES IN A CANCER CENTER

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INTRODUCTION:

Anti-CD19 CAR-T cell therapy has revolutionized the treatment of relapsed/refractory aggressive B-cell lymphomas, providing durable responses. In addition to major acute adverse effects such as cytokine release syndrome (CRS) and immune effector cell-associated neurotoxicity syndrome (ICANS), late hematological events like myelodysplastic syndrome (MDS) have been reported.[123] Although the causal mechanisms remain unclear, recent studies suggest a potential association with the number and intensity of prior treatments—especially autologous stem cell transplantation—and clonal predisposition linked to high-risk mutations.[145] The challenging management of persistent cytopenias and the risk of progression to acute myeloid leukemia (AML) increase negative outcomes and pose a clinical challenge.

OBJECTIVE:

To report two cases of secondary MDS following anti-CD19 CAR-T cell therapy for refractory B-cell lymphoma, highlighting clinical, laboratory, and disease course features.

METHODS:

Retrospective study based on medical record review, serial blood counts, bone marrow biopsies, karyotyping, and targeted gene panel analysis by next-generation sequencing (NGS).

RESULTS:

The first case involves an 80-year-old woman with diffuse large B-cell lymphoma refractory to three prior treatment lines, including autologous transplantation. She received anti-CD19 CAR-T cell therapy (axicabtagene ciloleucel) with complete response but developed persistent cytopenias after ten months. Bone marrow evaluation revealed high-risk MDS, with NGS identifying CBL and PPM1D mutations. She was treated with a hypomethylating agent but died from severe infectious complications. The second case is a 66-year-old woman with cutaneous diffuse large B-cell lymphoma (leg type), refractory to four treatment lines, including autologous transplantation, with subsequent CNS relapse. She received anti-CD19 CAR-T therapy (tisagenlecleucel) and achieved complete response.

Four months later, persistent cytopenias and transfusion dependence led to an AML diagnosis with a complex karyotype and 7q deletion, consistent with secondary AML. Despite treatment with a hypomethylating agent and BCL2 inhibitor, she died due to disease progression.

CONCLUSION:

The development of MDS after CAR-T therapy, though rare, is a severe complication with high morbidity and mortality. These cases underscore the need for prolonged monitoring in patients with persistent cytopenias, especially those heavily pretreated and with prior transplantation. The identification of high-risk mutations and cytogenetic abnormalities suggests a role for clonal hematopoiesis and cumulative damage in disease pathogenesis. Early diagnosis and long-term surveillance are key to timely intervention and improved outcomes.

KEYWORDS:

CAR T-cell therapy; Myelodysplastic syndrome; Late hematologic toxicity; Clonal hematopoiesis; Refractory B-cell lymphoma.

RISK MANAGEMENT IN CAR T CELL THERAPY: FMEA ANALYSIS AND STRATEGIES FOR SAFETY AND QUALITY THROUGHOUT THE PRODUCT LIFECYCLE

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INTRODUCTION:

CAR T cell therapy is a revolutionary treatment for hematologic malignancies, offering significant benefits for refractory patients. Given the therapy's complexity, robust risk management is essential to ensure patient safety, product quality, and regulatory compliance. Failure Mode and Effects Analysis (FMEA) provides a systematic approach for identifying and mitigating risks throughout the product lifecycle.

OBJECTIVE:

To develop and implement a continuous risk management framework for CAR T therapy, addressing all phases from patient selection to post-infusion monitoring to ensure safety, quality, and compliance.

METHODS:

FMEA was applied to identify failure modes, with risks quantitatively assessed by Severity (S), Occurrence (O), and Detection (D). Risk Priority Number (RPN = S×O×D) classified risks as acceptable (<100), moderate (100–199), or unacceptable (≥200). Priority was given to mitigating unacceptable risks through specific action plans.

RESULTS:

Process Stage	Critical Risk	RPN	Mitigation Actions
Patient Selection	Incorrect patient identification	250	ISBT 128, double verification, electronic vein-to-vein tracking, staff training, error simulations.
Lymphocyte Collection	Sample transport failure	200	Redundant carriers, contingency plans, audits, cold chain and emergency training.
Product Release	Non-conforming product release	300	Electronic criteria verification, multi-level review, non-conformity escalation, quality culture.
Product Infusion	CRS and neurotoxicity (ICANS)	350	Certified centers, patient education, 24/7 medical support, pharmacovigilance, real-world data use.
Product Infusion	Vein-to-vein traceability failure	420	Blockchain-based systems, rigorous validation, regular audits, clear data governance policies.

CONCLUSION:

An FMEA-based risk management plan for CAR T therapy ensures critical risk identification and targeted mitigation, promoting patient safety, product quality, and regulatory compliance. Continuous monitoring and plan updates support adaptation to evolving regulations and operational challenges, advancing safe and effective cellular therapies in Brazil.

KEYWORDS: CAR T cell therapy, risk management, FMEA.

FIGURE 1 – Leukapheresis Flow

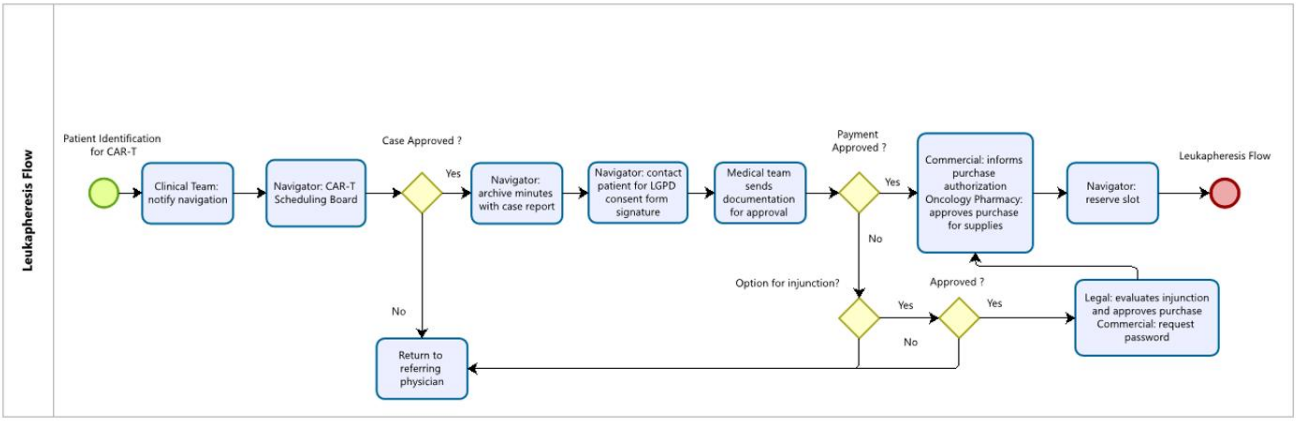


FIGURE 2 – Limphocyte Collection

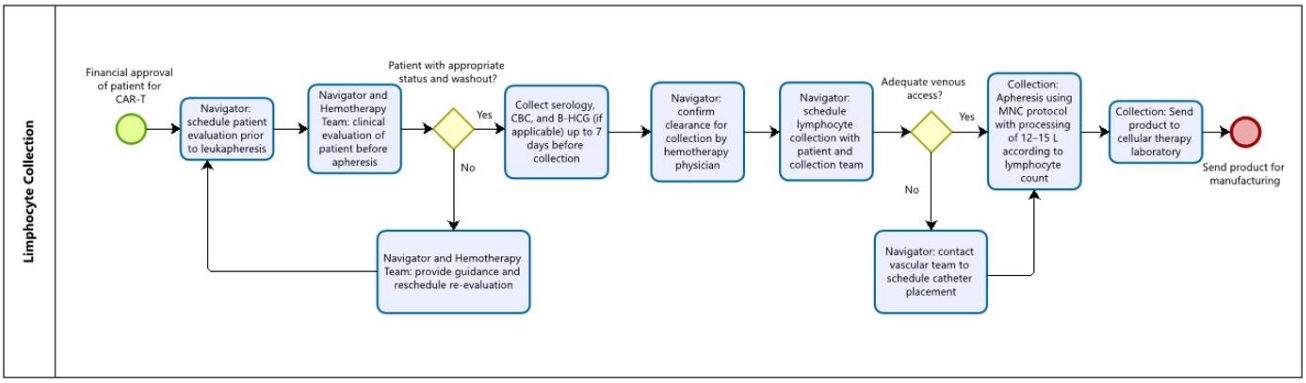
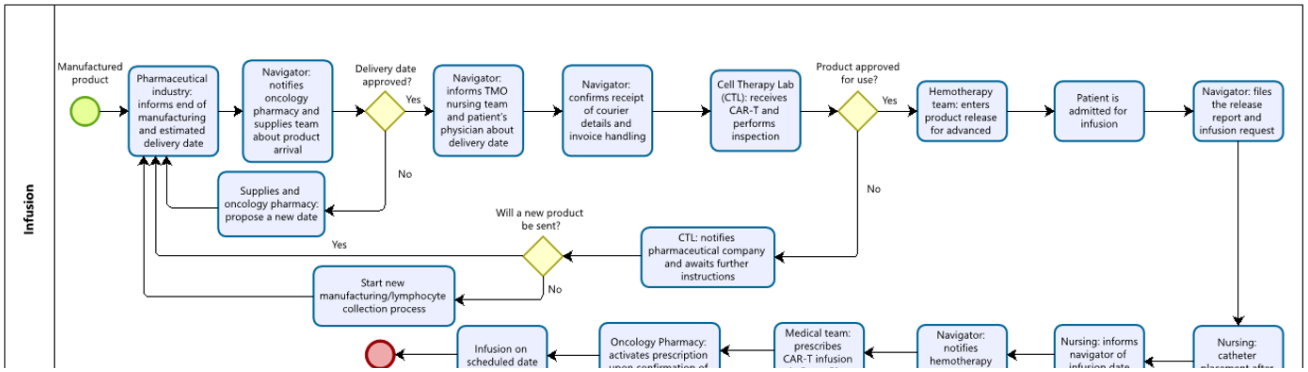


FIGURE 3 - Infusion



SECOND PRIMARY MALIGNANCY AFTER COMMERCIAL CHIMERIC ANTIGEN RECEPTOR T CELL THERAPY FOR ACUTE LYMPHOBLASTIC LEUKEMIA: A CASE REPORT

Bianca Batista Cal[1,3], Giovanna Alves Peruzini[1,3], Luciana Magalhães Brandão[1,3], Matheus de Lima Garcia[1,3] Júlia Aith Balthazar[1,3], Nina Maia Santana[1,3], Ana Costa Cordeiro[1,3], Vanessa dos Anjos Bovolenta[1,3], Marina de Mattos Nascimento[1,3], Marjorie Vieira Batista[1,2], Jayr Schmidt Filho[1,3]

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INTRODUCTION:

Although the use of chimeric antigen receptor (CAR) T-cell therapy is increasing worldwide, data on long-term toxicities, including secondary neoplasms, remain limited. Current literature reports secondary hematologic neoplasms as the most frequent entities associated with CAR-T, with T-cell malignancies predominating, followed by solid tumors and non-melanoma skin cancers. Notably, the FDA Adverse Event Reporting System (FAERS) has identified a statistically higher incidence associated with axicabtagene ciloleucel (axi-cel) and tisagenlecleucel (tisa-cel), with the latter showing a more pronounced association. However, conclusive evidence linking viral vector integration to secondary tumorigenesis remains elusive.

OBJECTIVES:

To report a case of a secondary neoplasm following CAR-T cell therapy for a relapsed/refractory acute lymphoblastic leukemia (ALL), contributing to the growing body of evidence on long-term toxicities of cellular therapies.

RESULTS:

An 18-year-old male was diagnosed in 2021 with Philadelphia-like B-cell ALL, refractory to frontline therapy (BFM 2009) and subsequent treatment with Blinatumomab plus Imatinib. Successful salvage therapy with Inotuzumab, Dasatinib, and Venetoclax was followed by a fully matched related allogeneic bone marrow transplantation in 2022. The post-transplant period was complicated by graft-versus-host disease and opportunistic infections, all of which were successfully managed. On day +265 post-transplant, relapse of ALL with meningeal involvement was diagnosed by positive cerebrospinal fluid analysis, prompting referral for CAR-T cell therapy. In April 2024, he received lymphodepletion with fludarabine/cyclophosphamide, followed by tisa-cel infusion (1.3 × 10^8 viable CAR-T cells). No cytokine release syndrome (CRS) or immune effector cell-associated neurotoxicity syndrome (ICANS) was observed. However, on day +16 post-infusion, he developed painless edema of the right upper limb. Ultrasound revealed a solid subcutaneous nodule. By day +60, the lesion had progressed, and biopsy confirmed a TFE3-positive sarcoma. However, subsequent analysis revealed a malignant neoplasm with a xanthomatous pattern and no evidence of TFE3 gene rearrangement, as assessed by fluorescence in situ hybridization. The patient underwent surgical resection of the lesion, with clear margins achieved and is currently under surveillance.

CONCLUSION:

The experience of our center aligns with existing literature suggesting a low but non-negligible incidence of therapy-related neoplasms post CAR-T therapy. While disease relapse remains the leading cause of mortality in this population, our case underscores the importance of long-term surveillance for secondary malignancies, particularly considering the potential role of viral vector integration. Further studies are needed to elucidate whether these events reflect CAR-T-specific oncogenicity or cumulative treatment-related effects.

KEYWORDS:

CAR-T, Secondary malignancy, Immune-targeted therapies.

TRANSFUSION PROFILE IN PATIENTS WITH MULTIPLE MYELOMA UNDERGOING FRESH AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION

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INTRODUCTION:

Hematopoietic stem cell transplantation (HSCT) is a highly complex procedure and patients undergoing this treatment option require continuous laboratory monitoring to assess the need for transfusions, since there is a period of pancytopenia until the transplanted stem cells start producing new blood cells.

OBJECTIVE:

To analyze the number and timing of packed red blood cells (RBC) and platelet concentrate (PC) transfusions received per patient during hospitalization for autologous HSCT. Method: Retrospective study describing the transfusion profile of patients with multiple myeloma who underwent fresh autologous HSCT at our institution from December 2021 until April 2025. In this study, it were considered the transfusions received from the mobilization of hematopoietic stem cells (HSC) until hospital discharge after engraftment. Data were obtained from the software used by the blood bank and from the hospital's electronic medical records.

RESULTS:

Transfusion data from the 62 transplanted patients were analyzed, the majority of whom were male (66.1%; n=41) and with a mean age of 58 years (SD=9.6; 31 to 73 years). Out of these 62 patients, only 3 patients (4.8%) did not require RBC or PC transfusion during hospitalization. Considering the transfused patients, 35.4% (n=22) received RBCs and 95.1% (n=59) received PC transfusion. RBC transfusions ranged from 1 to 6 units, with most patients receiving only 1 unit (54.5%; n=12), and the first transfusion occurred between 7 and 10 days after HSC infusion (63.6%, n=14). PC transfusions included both single-donor platelets and pooled platelets. In total, 59 patients received platelet transfusions, ranging from 1 to 5 transfusions during hospitalization, and of these 59 patients, 62.7% (n=37) received only one transfusion. The first platelet transfusion occurred between days 5 and 10 after infusion in all 59 transfused patients, with the highest number of transfusions occurring on days 7 and 8 post-infusion.

CONCLUSIONS:

Patients undergoing HSCT should be monitored by the blood bank staff, since most of these patients require transfusion support. RBC transfusions are not as frequent as PC transfusions, and patients who received more RBC units were patients with anemia prior to transplantation or who were already undergoing renal replacement therapy. Regarding platelet transfusion, blood banks need to ensure the availability of platelets, especially between days 5 and 10 after infusion, which can be challenging considering the short PC shelf life of only 5 days. Blood banks also need to ensure that the available RBC and PC units are ABO compatible, in addition to being irradiated and leukocyte-depleted especially in these first two weeks following infusion, but ideally during the first three months post HSCT.

KEYWORDS: hematopoietic stem cell transplant, multiple myeloma, blood transfusion.

TUMOR FLARE REACTION FOLLOWING TISAGENLECLEUCEL (KYMRIAH®) INFUSION IN A PATIENT WITH DLBCL: A CASE REPORT AND ADVERSE EVENT

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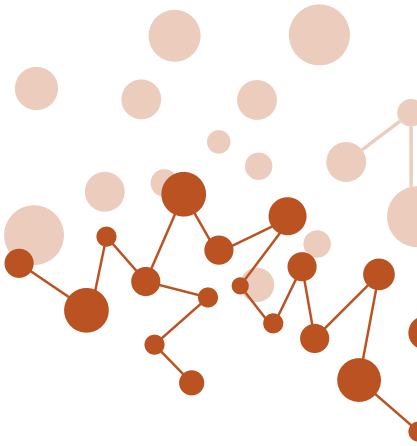
Diffuse large B-cell lymphoma (DLBCL) is the most common aggressive non-Hodgkin lymphoma, and the prognosis for patients with relapsed/refractory (R/R) disease remains challenging. Anti-CD19 CAR-T therapy, such as tisagenlecleucel (Kymriah®), is an approved option for R/R DLBCL, but adverse events like tumor flare reaction (TFR) require careful attention. We report the case of a 45-year-old male was diagnosed with DLBCL (germinal center subtype, BCL2/MYC double expressor, stage IIXEA) in August 2022. He initially received R-DA-EPOCH but experienced an early relapse. Salvage therapy with R-DHAP failed to induce a response. Subsequently, he received VIPOR-P (4 cycles) followed by radiotherapy, achieving a partial response and becoming eligible for CAR-T therapy. Lymphodepletion with fludarabine and cyclophosphamide (days -7 to -5) was followed by infusion of tisagenlecleucel (5.7×10^8 cells) on day 1. On day +2, the patient developed fever, prompting blood cultures and initiation of cefepime. Due to persistent fever, tocilizumab was administered on day +3 for prolonged grade 1 cytokine release syndrome (CRS). By day +4, the patient presented with abdominal pain and edema of the lower limbs and face. Imaging showed tumor enlargement, consistent with TFR. Symptoms were resolved spontaneously without corticosteroid use. PET-CT on day +30 confirmed complete metabolic response. TFR is a paradoxical inflammatory reaction characterized by transient clinical/radiological worsening post-infusion, associated with lymphocyte expansion. Markers such as elevated LDH and cytokines (without CRS correlation) may aid in diagnosis. For tumors near critical structures, vigilance is required for compressive symptoms (neurological or vascular). Management may include systemic corticosteroids, surgical intervention, or radiotherapy if necessary. TFR is a clinically significant adverse event that requires early recognition and appropriate management. Corticosteroids are effective without compromising CAR-T efficacy, underscoring the importance of multidisciplinary monitoring.

KEYWORDS: tumor flare reaction, CAR-T cell therapy, Diffuse Large B-Cell Lymphoma

ACADEMIC LEAGUES



ARTIFICIAL INTELLIGENCE IN RISK STRATIFICATION OF ONCO-HEMATOLOGICAL DISEASES AND HEMATOPOIETIC CELL TRANSPLANTATION

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INTRODUCTION:

Risk stratification is an essential component of care in onco-hematology, guiding therapeutic decisions, transplantation indication, and prognostic discussions. However, the clinical and biological heterogeneity of these diseases often limits the effectiveness of traditional models, especially within intermediate-risk groups. In this context, Artificial Intelligence (AI) has gained prominence as a promising tool to refine prognostic assessment, offering greater precision and clinical sensitivity.

OBJECTIVE:

To analyze how the application of Artificial Intelligence, particularly through machine learning techniques, has contributed to improving risk stratification in onco-hematological diseases.

A narrative literature review was conducted across PubMed, Scopus, Web of Science, and Scielo databases, covering the period from 2018 to 2024. Descriptors included: artificial intelligence, machine learning, risk stratification, onco-hematological diseases, prognostic models, myelodysplasia, and myelofibrosis. Studies applying AI algorithms to clinical, laboratory, and/or genomic data, with robust statistical validation and a focus on practical applicability, were prioritized.

RESULTS:

Multiple studies demonstrate that AI, through machine learning algorithms, can identify patterns and relationships among variables that conventional models fail to capture. This has enabled the development of more sensitive scoring systems capable of providing individualized predictions even with basic clinical data. One example is the AIPSS-MF, developed for patients with myelofibrosis, which showed superior performance compared to the classic IPSS without using molecular information. Besides improving accuracy, these models maintain simplicity and broaden applicability in resource-limited centers. AI has also been useful in reclassifying patients initially grouped imprecisely by traditional systems, which may have direct therapeutic implications.

CONCLUSIONS:

Artificial Intelligence has established itself as a strategic ally in risk stratification in onco-hematology. Its judicious use can not only complement existing models but also transform them, making clinical reasoning more sensitive to the nuances of each case. The current challenge is to validate these models in different contexts and ensure their ethical, interpretable, and effective integration into routine care.

CURRENT STATE AND PERSPECTIVES ON THE USE OF MESENCHYMAL STROMAL CELLS FOR STEROID-REFRACTORY GVHD

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INTRODUCTION.

GvHD is a severe complication of allogeneic HSCT, resulting from the donor immune response against recipient tissues. In up to 50% of cases, acute GvHD becomes steroid-refractory (SR), associated with high mortality and limited effective treatments. In this context, mesenchymal stromal cells (MSCs) have emerged as a promising therapeutic alternative due to their strong immunomodulatory properties, possibly mediated by cytokines such as TGF-β, IDO, PGE2, and by the induction of Treg cells. Despite their potential, challenges such as variability among cell sources, lack of standardization, and heterogeneous clinical responses still hinder their widespread clinical use.

OBJECTIVES.

This study provides a critical review of MSC therapy for SR-GvHD. The efficacy for both aGvHD and cGvHD and the profile of best responding patients is reviewed. The study intends to serve as an update and synthesis of important findings.

METHOD.

Searches in Pubmed and Cochrane library using keywords "GvHD", "stromal cell" and "MSC" in varying combinations and synonyms, followed by selection of the most relevant publications by the authors have yielded 20 studies.

RESULTS.

Usage of MSCs has been repeatedly demonstrated as safe, but the quality of the evidence supporting its efficacy is low and appears to vary depending on patient profiles. A systematic review by Fisher et al. synthesized favorable evidence for the prophylactic use of MSCs in reducing the number of cGvHD cases but found no benefits of the intervention in reducing mortality among transplant recipients. A RCT conducted by Zhao et al. showed a significant increase in the overall response (OR) of recipients with SR-GvHD treated with MSCs plus basiliximab and a calcineurin inhibitor compared to controls receiving only basiliximab and the latter options. However, negative results are still present in some trials, which may be explained by heterogeneity of MSCs. Younger age groups with SR-GvHD show better overall response (OR) to MSC therapy than adults. More predictors of MSC response have not been fully validated but appear to include a pro-inflammatory environment prior to infusion; lower levels of GvHD damage markers; and higher MSC doses are associated with higher OR. Patients with grade II aGvHD had better OR in Zhao et al.'s study, but the difference in OR between controls and MSC recipients was greater for grade IV. On the other hand, CR and OS are more strongly associated with earlier stages in most studies.

CONCLUSION.

MSCs are a promising alternative in SR-GvHD by modulating immunity. However, challenges such as lack of standardization, and clinical variability limit their adoption. Overcoming these barriers requires the discovery predictive biomarkers, and multicenter studies. The consolidation of cell therapy in clinical practice depends on solid evidence supporting its effective use and the situations in which to use it.

KEYWORDS:

GVHD, Mesenchymal Stem Cells, HSCT.

TABLE 01 - Lorem ipsum spreadsheet

Column 1	Column 2	Column 3	Column 4	Column 5
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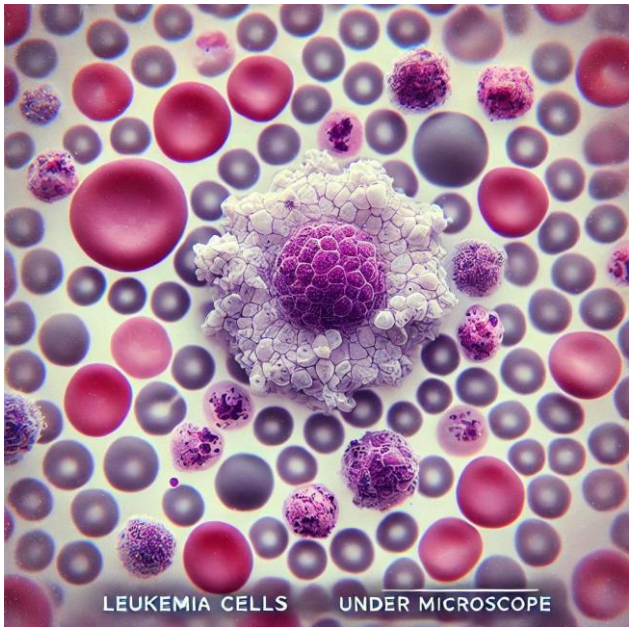
FIGURE 1



EX VIVO EXPANSION FOR HEMATOPOIETIC STEM CELL TRANSPLANTS: A LITERATURE REVIEW

Fernando Silva de Oliveria[1] , Gabriel Lucas de Souza Cordeiro[1], Ângelo Antônio Silva Lima[1], Alessandra Rocha Ribeiro Souto[1], João Victor Macêdo da Cunha[1], Maria Eduarda Melo de Oliveira Castro[1]

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INTRODUCTION:

The ex vivo expansion of hematopoietic stem cells (HSCs) represents a promising approach to overcome quantitative limitations of available cell units for transplantation, particularly in the context of umbilical cord blood. Several clinical trials have demonstrated the potential of these expanded cells to promote faster engraftment, effective hematologic recovery, and clinical safety. Although most research focuses on cord blood-derived HSCs, alternative sources such as bone marrow and peripheral blood have also been investigated.

OBJECTIVE:

To review the recent scientific literature on the clinical application of ex vivo expansion of HSCs, with an emphasis on clinical trials and reviews reporting outcomes related to safety, efficacy, and therapeutic feasibility in hematopoietic transplants.

MATERIALS AND METHODS:

This is a literature review conducted in the PUBMED database, covering the period from 2015 to 2025, using the descriptors: "Hematopoietic Stem Cell" AND "Expansion" AND "Ex vivo." The search yielded 70 articles. Case reports, case series, and restricted-access articles were excluded. Three review studies and three clinical studies with direct applicability were included. The analysis considered the type of study, source of HSCs, expansion approach, and clinical outcomes.

RESULTS:

The reviewed studies indicate that ex vivo expansion is feasible and effective, primarily with umbilical cord blood cells, using agents such as StemRegenin-1 (SR-1), nicotinamide, and UM171. Wagner et al. (2016) demonstrated in a phase I/II trial that the use of SR-1-expanded HSCs enables efficient engraftment with a single graft, with a favorable safety profile. Reviews indicate that these approaches result in accelerated hematologic recovery and a potential reduction in graft-related mortality. There is also emphasis on strategies for cellular homing and enhancement of the hematopoietic microenvironment. Although less frequently, the use of expanded HSCs from bone marrow and peripheral blood has been explored in specific contexts, such as immune reconstitution following chemotherapy.

CONCLUSION:

Current literature demonstrates significant progress in the transition of ex vivo HSC expansion from the laboratory to clinical application, especially with cord blood cells. Clinical trials have shown the potential of these expanded cells in achieving efficient engraftment with adequate safety. Alternative sources such as bone marrow warrant further investigation. The consolidation of these strategies could significantly improve access to and effectiveness of hematopoietic transplants, particularly in populations with limited access to compatible donors.

KEYWORDS: Hematopoietic Stem Cells; Ex Vivo Expansion; Cord Blood Transplantation.

HAPLOIDENTICAL ALLO-HSCT FOR PRIMARY MYELOFIBROSIS, PROGNOSTIC RECLASSIFICATION AND U2AF1 MUTATION: A CASE REPORT

Vitor Cavalcante Monici[1], Fernando Silva de Oliveira[1], Gabriel Lucas de Souza Cordeiro[1], Ana Carolina Araújo de Oliveira[1], João Victor Macêdo da Cunha[1], Jose Alberto Souza Abdon[2], Flávia Dias Xavier[1,2]

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INTRODUCTION:

Primary myelofibrosis (PMF) is a chronic myeloproliferative neoplasm. Prognostic stratification is essential to guide allogeneic hematopoietic stem cell transplantation (allo-HSCT), the only curative therapy.

CASE PRESENTATION:

AG, 58-year-old male, was diagnosed with PMF in Nov 2024. Symptoms began in Jul 2024 (asthenia, body aches, fever, night sweats) with progressive cytopenias (platelets: 69,000/mm^3; Hb: 11.8 g/dL). Bone marrow biopsy revealed 50% cellularity and grade 3 fibrosis. Initial DIPSS showed intermediate-1 risk (median survival: 14.2 years). DIPSS-plus reclassified as intermediate-2 (2.9 years), and MIPSS70 assigned high risk (score >5; 3.1 years), corroborated by MIPSS70-plus v2.0. GIPSS also indicated high risk (≥2 points; 4.2 years), pending karyotype. NGS (Oct 11, 2024) identified U2AF1 mutation (VAF 2.7%), JAK2 V617F wild-type. This mutation and scoring convergence supported an aggressive approach. Ruxolitinib (10 mg BID) was started on Nov 22, 2024, as a bridge to allo-HSCT. The patient was eligible and listed for transplant. In Dec 2024, alfaepoetin (Eprex weekly from Dec 3) was initiated due to transfusion-dependent anemia. No significant splenomegaly was present requiring further intervention. Allo-HSCT occurred on Jan 21, 2025, from a haploidentical related donor (niece). Conditioning was Fludarabine-Busulfan (FluBu2) with total body irradiation (TBI). GvHD prophylaxis included post-transplant cyclophosphamide (PTCy), mycophenolate mofetil (MMF), and cyclosporine. Neutrophil engraftment occurred on D+17 (Feb 7, 2025). Initial post-transplant course included grade 1 mucositis. Platelet engraftment was ongoing as of Feb 24, 2025.

DISCUSSION:

Allo-HSCT decisions in PMF benefit from comprehensive prognostic tools. This case illustrates how advanced models (DIPSS-plus, MIPSS70/v2.0, GIPSS) and molecular data (U2AF1 mutation) upgraded the DIPSS classification, supporting early transplant. U2AF1 mutations are adverse markers and influence eligibility. Reduced-intensity conditioning (RIC), such as FluBu2, lowers non-relapse mortality (NRM) while preserving disease control—especially relevant for older patients. Studies report ~60% 5-year OS with RIC and NRM of 20–30%. TBI adds efficacy in myelofibrosis settings. Haplo-HSCT with PTCy is a viable option when matched donors are unavailable. Literature reports engraftment rates ~76%, though graft failure impacts survival. This case supports the feasibility of haplo-HSCT in high-risk PMF.

CONCLUSION:

This case underscores the role of modern prognostic tools and molecular profiling in PMF management. U2AF1 mutation and risk scores guided the decision for allo-HSCT, successfully performed using haploidentical FluBu2/TBI conditioning with PTCy. Although early outcomes were favorable, long-term follow-up remains key. Emerging therapies (e.g., ruxolitinib plus navitoclax, pelabresib, bomedemstat) are promising, but their role relative to transplant requires further study.

KEYWORDS: Primary Myelofibrosis; Haploidentical Transplantation; U2AF1 Mutation; Prognostic Scoring Systems; Post-Transplant Cyclophosphamide.

HLA-IDENTICAL RELATED DONOR TRANSPLANT IN A PATIENT WITH APLASTIC ANEMIA, LARGE GRANULAR LYMPHOCYTIC LEUKEMIA, AND PERIPHERAL T-CELL LYMPHOMA: A CASE REPORT

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INTRODUCTION:

Aplastic anemia (AA) is a bone marrow failure syndrome often linked to autoimmune mechanisms. Large granular lymphocytic leukemia (LGL), characterized by clonal proliferation of cytotoxic T lymphocytes, is associated with autoimmune cytopenias like pure red cell aplasia (PRCA) and may underlie some AA cases. Rarely, LGL progresses to peripheral T-cell lymphomas (PTCL), reflecting malignant clonal evolution. This clinical sequence highlights complex immune-neoplastic interactions. Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is a potentially curative option for refractory or relapsed PTCL after conventional therapy failure.

METHODS:

We conducted a case report in accordance with ethical standards to explore the use of allo-HSCT in a patient with severe AA, LGL, and PTCL.

CASE REPORT:

A 52-year-old male (RURR) was diagnosed with idiopathic AA in November 2023, initially managed with immunosuppressive therapy. In March 2024, LGL and cutaneous/peripheral T-cell lymphoma (C84) were confirmed by T-cell receptor gamma and beta gene rearrangements. Methotrexate and prednisone were discontinued due to disease progression and encephalitis/hepatitis development. Cladribine and ruxolitinib failed to yield response. The patient developed hepatic iron overload, proteinuria, and sensorineural hearing loss. Deferasirox (17 mg/kg) was started in November 2024, worsening renal function. In March 2025, the patient underwent allo-HSCT from an HLA-identical sibling donor with a conditioning regimen of fludarabine, melphalan, and antithymocyte globulin (FluMel + ATG). Neutrophil engraftment occurred by day +14. Graft-versus-host disease (GVHD) prophylaxis included reduced-dose post-transplant cyclophosphamide and cyclosporine A. Complete donor chimerism was achieved, and the patient remains under outpatient monitoring.

DISCUSSION:

The sequential occurrence of AA, LGL, and PTCL, though rare, represents a progressive clonal spectrum driven by overlapping immunopathogenic mechanisms. Given refractoriness to immunosuppressants and chemotherapy, allo-HSCT was appropriate, consistent with current recommendations for relapsed/refractory PTCL. Reduced-intensity conditioning (FluMel + ATG) was selected due to prior toxicity and organ dysfunction. Similarly, GVHD prophylaxis with reduced-dose cyclophosphamide and cyclosporine A balanced engraftment with complication risk. Sustained monitoring remains vital, given late complication risks.

CONCLUSION:

This case illustrates potential clonal progression from AA to LGL and PTCL and reinforces allo-HSCT as a viable strategy in refractory hematologic diseases. Reduced-intensity regimens and individualized GVHD prophylaxis can optimize outcomes in complex, comorbid patients. Long-term surveillance is essential for managing late effects and advancing understanding of rare clonal hematologic disorders.

KEYWORDS: Aplastic Anemia; Large Granular Leukemia; Allogeneic transplantation.

MODIFICATION OF THE INTESTINAL MICROBIOME AND BONE MARROW TRANSPLANTATION OUTCOMES

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INTRODUCTION:

Bone Marrow Transplantation (BMT) is a special therapy used for various diseases, be they malignancies or hematopoietic disorders. Transplants may be autologous or allogeneic, the latter predisposing the recipient to graft vs host disease (GVHD). Preparation for BMT is multi-faceted and involves bone marrow ablation, antibiotic administration, special diets with low microbial load, and isolation in laminar-airflow rooms. These measures prepare for the state of immunosuppression the recipient undergoes after myeloablation and thus seek to avoid any possible infection. Such transplantation regimen invariably alters the microbiota, a diverse ecosystem of microbes, exerting several important functions, such as immune system modulation and protection of the digestive tract. The loss of microbiota diversity in the course of BMT preparation thus causes great changes in the recipient that lead to several negative effects, such as increased risk of infections, of inflammation, higher rates of GVHD, and, overall, higher morbidity and mortality occur in patients with heightened gut dysbiosis.

OBJECTIVE:

This review analyzes how the gut microbiome affects BMT outcomes and complications.

METHODS:

This is a literature review of the MedLine database (2018–2025) using terms related to intestinal microbiome and bone marrow transplantation. After applying inclusion criteria (peer-reviewed, English, empirical, relevant, with available abstracts), six studies were selected and analyzed.

RESULTS:

Gut microbiome integrity is strongly associated with allogeneic BMT, being demonstrated a higher overall mortality (cohort 1: (n=354 and n=350); adjusted hazard ratio, 0.71; 95% confidence interval [CI], 0.55 to 0.92; cohort 2: (n= 87 and n=92); adjusted hazard ratio, 0.49; 95% CI, 0.27 to 0.90) and increased mortality in patients with low microbiota diversity. Consequently, new approaches are being tested to enable better outcomes. Prebiotics and probiotics seem to be more effective than placebo in preventing severe forms of acute GVHD, although they have not improved overall survival or the occurrence of chronic GVHD. Another potential strategy is fecal microbiota transplantation; however, it has not demonstrated statistical significance in preventing infections (infection rate ratio, 0.83; 95% CI, 0.48 to 1.42; P = .49) or reducing complications.

CONCLUSION: BMT preparation induces dysbiosis in the microbiome of patients, who show a greater risk of infections by opportunistic pathogens, of GVHD, and increased transplant mortality. While therapeutic strategies such as the use of probiotics, prebiotics, and fecal microbiota transplantation show modest benefits, future studies should focus on linking the gut microbiota with BMT outcomes, emphasizing treatments focused on personalization and microbiological risk stratification.

KEYWORDS: Bone marrow transplantation; Microbiota; Graft vs host disease.

MULTIPLE MYELOMA: THERAPEUTIC WINDOW FOR BONE MARROW TRANSPLANTATION

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INTRODUCTION

Representing nearly 10% of hematological pathologies, Multiple Myeloma (MM) is a neoplasm disorder which affects plasma cells, resulting in abnormal and clonal proliferation in the bone marrow as well as excessive production of antibodies. The current recommended therapy is bone marrow transplantation (BNT), more specifically the autologous stem cell transplantation (ASCT) and, for eligible patients, it is recommended 3 to 6 cycles of chemotherapy induction performed before transplantation.

OBJECTIVE

To analyze the therapeutic window for bone marrow transplantation in patients with multiple myeloma.

METHOD

Integrative review on the therapeutic window for BMT in patients with MM. The literature search was carried out in the PubMed database using the descriptors "Multiple Myeloma", "Bone Marrow" and "Transplantation". 24 articles found passed the selection criteria (primary articles, related to the research objective, published in the last 5 years), remaining 6 that were selected after full reading for the final analysis.

RESULTS

Charalampous et al. 2022 evaluated 1,055 patients undergoing ASCT after induction therapy and chemotherapy. The median time from the last cycle of induction and transplantation was 33 days. The study concluded that delaying the ASCT results inferiorly considering disease-free time and overall survival rate. Ricciuti al. 2020 indicates a similar survival rate with early transplantation (after four cycles of induction therapy) compared to late transplantation (at the time of relapse as rescue therapy). The study points out that low-risk patients can choose to delay transplantation without a significant increase in unfavorable outcomes. Pasvolsky et al. 2024 showed that only 15% of the patients submitted to ASCT analyzed progressed with a positive long-term response. Early age, lower probability of high-risk cytogenetics, grade 1 staging and association with post-transplant maintenance therapy were favorable to this outcome. Devarakonda et al. 2021 showed that the addition of post-ASCT maintenance therapy improved the median disease-free time from 23-27 months to 47-53 months and the median survival rate from 3–5 years to 7–10 years. Rajkumar et al. 2024 indicated maintenance with lenalidomide for low-risk patients, combined bortezomib and lenalidomide for high-risk patients, and a specific triple regimen for cases of relapse. Therapy with chimeric antigen receptor T cells (CAR-T) and bispecific antibodies are options that have shown to deepen the response to the minimal residual disease state, aiming to prolong disease-free time.

CONCLUSION

Adequate identification of the therapeutic window, performing early ASCT after induction therapy and before disease progression, combined with treatment personalization, considering favorable immunological profiles, use of immunomodulators and specific antibodies, contributes significantly to the success of ASCT and prolonged control of the disease.

PROGNOSIS OF ACUTE LYMPHOBLASTIC LEUKEMIA IN ADULTS: NEW STRATEGIES FOR RISK STRATIFICATION

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INTRODUCTION:

Acute lymphoblastic leukemia (ALL) is a disease that occurs in children and has a 5-year survival prognosis of approximately 85%. However, this same disease has a worse prognosis in adults. Factors such as advanced age, genetic alterations like the Philadelphia Chromosome (Ph), hypodiploidy, hyperploidy and KMT2A rearranged ALL, leukocytosis and minimal residual disease values are the main factors for determining the prognosis in adults. Considering this, it is necessary to study the importance of the risk stratification according to the specific factors in these patients. Objectives: To explore the prognostic factors that guide the risk stratification in ALL in adults based on the guiding question: "What criteria determine the risk and prognosis of ALL in adults?".

METHODS:

A literature review was conducted with 7 studies published in the last 5 years, published on PubMed, ScienceDirect, European Journal of Cancer and Springer Nature Link databases. The descriptors were: acute lymphoblastic leukemia, prognosis and adults. Statistical analyses of the articles included chi-square tests, Cox regression, Fisher's exact test and the Kaplan- Meier curve, some articles show a significance level of 5%.

RESULTS:

In addition to the risk stratification factors mentioned, it is possible that advances in genetic sequencing technology will lead to the inclusion of the IKZF1 gene mutation in the risk stratification parameters,after all, it is a factor present in approximately 70% of patients with Ph-positive-like ALL and indicates an unfavorable prognosis. This gene, when expressed in dominant-negative isoforms, is associated with the development of ALL. In addition, alterations in the genes that encode the Janus kinase/signal transducer and activator of transcription (JAK/STAT) signaling pathway. Mutations, especially in the JAK1 gene, are related to ALL, approximately 50% of cases of Ph-positive-like ALL, 6.5 to 27% of cases of T-ALL and 1.5% of cases of B-ALL. Defects in this gene lead to dysfunction of hematopoietic function mediated by inflammatory factors, which translates into a poor prognosis for its carriers.

CONCLUSION:

ALL in adults has a more aggressive and less favorable prognosis, therefore, specific risk stratification is essential for the effectiveness and monitoring of therapy. In this sense, stratification encompasses several clinical, laboratory and genetic aspects, and, with advances, it is possible that the IKZF1 gene mutation and defects in the JAK/STAT signaling pathway may be added. On the other hand, the molecular tests discussed are expensive and require greater infrastructure, but they can direct treatments, helping to increase survival and mitigate relapses in patients. Thus, they can be beneficial, even though they present challenges for the health system in relation to their implementation. Finally, these advances can increase positive results, diversify therapeutic measures and improve clinical practice.

KEYWORDS: Precursor Cell Lymphoblastic Leukemia-Lymphoma; Genetic Risk Score; Adult