

Hematopoietic cell transplantation outcomes in public centers in Brazil: analysis of the Brazilian Registry of Hematopoietic Cell Transplantation and Cellular Therapy, 2012–2024

Anderson João Simione^{1*} , Cinthya Correa da Silva² , Nelson Hamerschlak² , Afonso Celso Vigorito³ , Heliz Regina Alves das Neves⁴ , Adriana Seber^{5,6,7} , Carmem Maria Sales Bonfim⁸ , Vergilio Antonio Rensi Colturato¹ , Samir Kanaan Nabhan⁴ , Vanderson Geraldo Rocha⁹ , George Mauricio Navarro Barros¹⁰ , Decio Lerner¹¹ , Liane Esteves Daudt¹² , Juliana Folloni Fernandes¹³ , Marcos Paulo Colella³ , Alexandre Silverio¹⁴ , Rodolfo Daniel de Almeida Soares¹⁵ , Gustavo Machado Teixeira¹⁶ , Victor Gottardello Zecchin⁵ , Abrahao Elias Hallack Neto¹⁷ , Rony Schaffel¹⁸ , Rodolfo Froes Calixto¹⁹ , Simone de Castro Resende Franco²⁰ , Gianne Donato Costa Veloso²¹ , Vaneuza Araújo Moreira Funke⁴ , Fernando Barroso Duarte²² 

1. Hospital Amaral Carvalho  – Jaú (SP), Brazil.

2. Hospital Israelita Albert Einstein  – São Paulo (SP), Brazil.

3. Universidade Estadual de Campinas  – Hemocentro – Campinas (SP), Brazil.

4. Universidade Federal do Paraná  – Hospital de Clínicas –Curitiba (PR), Brazil.

5. Universidade Federal de São Paulo  – Instituto de Oncologia Pediátrica – São Paulo (SP), Brazil.

6. Grupo de Apoio ao Adolescente e à Criança com Câncer  – São Paulo (SP), Brazil.

7. Hospital Samaritano de São Paulo  – São Paulo (SP), Brazil.

8. Hospital Pequeno Príncipe – Curitiba (PR), Brazil.

9. Universidade de São Paulo  – Hospital das Clínicas – São Paulo (SP), Brazil.

10. Hospital de Câncer de Barretos  – Barretos (SP), Brazil.

11. Instituto Nacional do Câncer  – Rio de Janeiro (RJ), Brazil.

12. Hospital de Clínicas de Porto Alegre  – Porto Alegre (RS), Brazil.

13. Universidade de São Paulo  – Faculdade de Medicina – Instituto da Criança – São Paulo (SP), Brazil.

14. Centro de Pesquisas Oncológicas  – Florianópolis (SC), Brazil.

15. Natal Hospital Center – Natal (RN), Brazil.

16. Universidade Federal de Juiz de Fora  – Serviço de Transplante de Medula Óssea – Belo Horizonte (MG), Brazil.

17. Universidade Federal de Juiz de Fora  – Hospital Universitário – Juiz de Fora (MG), Brazil.

18. Universidade Federal do Rio de Janeiro  – Hospital Universitário Clementino Fraga Filho – Rio de Janeiro (RJ), Brazil.

19. Real Hospital Português  – Recife (PE), Brazil.

20. Hospital da Criança de Brasília José Alencar – Brasília (DF), Brazil.

21. Santa Casa de Montes Claros  – Montes Claros (MG), Brazil.

22. Universidade Federal do Ceará  – Hospital Universitário Walter Cantídio – Fortaleza (CE), Brazil.

*Corresponding author: ajsimione@hotmail.com

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ABSTRACT

This study provides a national overview of hematopoietic cell transplantation (HCT) outcomes in the Brazil's public healthcare network, using data consolidated in the Brazilian Registry of Hematopoietic Cell Transplantation and Cellular Therapy (RBTC-TC). A total of 5,923 first HCTs performed between 2012 and 2024 across 19 public centers were analyzed. Median follow-up among survivors reached 24.5 months, reflecting recent improvements in follow-up completeness and data reliability within the registry. Overall survival, relapse, and non-relapse mortality (NRM) rates were generally consistent with international benchmarks, despite the resource limitations characteristic of a publicly funded system. NRM was concentrated in the first year after allogeneic HCT, whereas relapse remained the predominant cause of late mortality across both autologous and allogeneic procedures. Acute graft-versus-host disease (GVHD) was more frequent among recipients of mismatched related and unrelated donors, while chronic GVHD occurred more often in matched related donor transplants. These findings highlight the complexity of transplant care in the public setting and underscore the importance of standardized data collection, integration across centers, and long-term surveillance to support continuous quality improvement. Strengthening national collaborative initiatives such as the RBTC-TC remains essential for guiding evidence-based strategies and improving patient outcomes throughout Brazil's public transplant network.

Keywords: Hematopoietic Cell Transplantation. Data Management. Unified Health System.

INTRODUCTION

A structured and comprehensive approach to monitoring hematopoietic cell transplantation (HCT) results is fundamental for improving clinical outcomes, promoting uniformity of care, and guiding health policies based on real-world evidence. In Brazil, the continuous growth and increasing sophistication of cellular therapies have created a clear demand for reliable national platforms capable of tracking procedures and long-term results. In response, a coordinated initiative was implemented to gather, verify, and harmonize HCT data across institutions, ensuring consistent and ongoing evaluation throughout public transplant centers¹.

The Center for International Blood and Marrow Transplant Research (CIBMTR), a partnership between the Medical College of Wisconsin and the National Marrow Donor Program (NMDP), has captured global HCT activity since its inception. Brazil has contributed to this initiative since 1989. A major milestone occurred in 2016, when the Brazilian Society of Cellular Therapy and Bone Marrow Transplantation (SBTMO) formalized a strategic partnership with the CIBMTR. This initiative created a structured national training program focused on improving data quality, enhancing completeness of reporting, and standardizing transplant documentation across centers, thereby substantially strengthening Brazil's participation in international registries¹.

As a direct result of these efforts, the Brazilian Registry of Hematopoietic Cell Transplantation and Cellular Therapy (RBTC-TC) was established², consolidating information from participating centers and publishing national outcome data annually through the Brazil Summary Slides³⁻⁷. This initiative has allowed unprecedented visibility into Brazil's transplant activity and outcomes, supporting benchmarking efforts and facilitating the adoption of best practices across institutions.

The Brazilian National Transplant System (SNT) has played a central role in supporting the consolidation, interpretation, and application of registry data. Recognizing the reliability of HCT registry information, the SNT has used these data to address specific informational needs of public transplant centers and to support strategic planning and policy development. This collaboration reflects a shared national commitment to transparency, continuous improvement, and the strengthening of Brazil's public transplant network.

This study aimed to present national data on HCT performed in public centers in Brazil, using information consolidated in the Brazilian RBTC-TC. The analysis was developed in collaboration with the Brazilian SNT, with the goal of providing a comprehensive overview of transplant activity, survival outcomes, treatment-related mortality, relapse rates, and graft-versus-host disease incidence. These findings are intended to support the SNT in developing a clinical and situational diagnosis of the public transplant network and to inform future health policy planning.

METHODS

Data sources

HCT data from Brazil are routinely entered into the CIBMTR database using FormsNet3, an electronic system with two-step authentication for all registered users. Once processed, cleaned, and coded, the information is redistributed to the SBTMO via the Data Back to Center (DBtC) tool, supporting continuous evaluation of outcomes at both center and national levels⁶.

Selection

Data were extracted from the CIBMTR portal through the DBtC tool, including 5,923 first HCTs carried out between 2012 and 2024. Among them, 3,035 were autologous procedures and 2,888 were allogeneic. The dataset represents activity from 19 public Brazilian centers that report their transplant information to the CIBMTR.

Only cases with complete information on transplant type, diagnosis, graft source, and at least one follow-up update were included in the analysis. Additionally, patients were required to have documented data on disease relapse and follow-up or event dates.

Detailed eligibility criteria are presented in Table 1.

Table 1. Inclusion and exclusion criteria for patient selection.

Selection criteria	Excluded	N
Transplants performed between 2012 and 2024		14,791
Public centers	4,847	9,944
First transplant	874	9,070
Exclusion		
Patients without follow-up data	832	8,238
Missing information on relapse	2,232	6,006
Missing date of relapse	83	5,923
Total		5,923

Source: Elaborated by the authors.

After extraction, the dataset was uploaded to Power BI Desktop (PBI), in which updated validation routines were applied to verify the total number of transplants and participating centers. These checks ensured alignment between the analyzed records and the information maintained in the registry.

Definitions and outcomes

- Patients were grouped into pediatric (0–17 years old) and adult (≥ 18 years old) categories;
- Allogeneic procedures were stratified according to donor relationship: matched related, mismatched related (including haploidentical and other single-mismatch relatives), and unrelated donors;
- Cell source was classified as bone marrow (BM), peripheral blood stem cells (PBSC), or umbilical cord blood (UCB)⁶.

Overall survival

Overall survival was estimated using the Kaplan–Meier method. The event was defined as death from any cause, and patients were censored at the date of last follow-up if alive. Median follow-up time and survival estimate at specific time points were calculated with 95% confidence intervals.

Non-relapse mortality

To evaluate non-relapse mortality (NRM), we applied cumulative incidence analysis with competing risks, treating relapse as the competing event⁸.

Relapse

The cumulative incidence of disease relapse was analyzed for both autologous and allogeneic transplants, considering death without relapse as a competing risk. Only patients with complete relapse information were included in this analysis.

ACUTE GRAFT-VERSUS-HOST DISEASE

The cumulative incidence of acute graft-versus-host disease (aGVHD) grades II–IV and III–IV was assessed among allogeneic HCT recipients, considering death without aGVHD as a competing event. Exclusion criteria included transplants performed before 2017 (as the date of aGVHD diagnosis became available only from 2017 onward), cases without information on aGVHD occurrence, missing date of aGVHD diagnosis, missing aGVHD grading, and erroneous records in which the reported date of aGVHD diagnosis was inconsistent with transplant or follow-up data⁹.

Chronic graft-versus-host disease

For the analyses of chronic graft-versus-host disease (cGVHD), the cumulative incidence of global and moderate/severe grades was evaluated among allogeneic HCT recipients, considering death without cGVHD as a competing risk. Cases without information on cGVHD occurrence, missing date of cGVHD diagnosis, missing cGVHD grading, or erroneous records in which the reported date of cGVHD diagnosis was inconsistent with transplant or follow-up data were excluded⁹.

Statistical analysis

Descriptive analyses were conducted to summarize the dataset. Categorical variables were reported as counts and percentages, whereas continuous variables were expressed as medians with their respective ranges. Overall survival was assessed using the Kaplan-Meier method. Cumulative incidence curves were generated to evaluate events in the presence of competing risks. All survival and competing-risk analyses were performed using R Statistical Software (version 4.4.1)⁹.

Ethical considerations

Ethical authorization to use the CIBMTR platform for research within the Brazilian Registry was granted by the national Institutional Review Board (IRB) in 2019 (CONEP CAAE: 65575317.5.1001.0071), under the responsibility of the principal investigator, Dr. Nelson Hamerschlak⁶.

RESULTS

Baseline characteristics and clinical outcomes are summarized below. Analyses were performed considering a median among survivor's follow-up of 25.4 months for the cohort. The completeness of follow-up, defined as the proportion of patients with updated follow-up information at each timepoint—90% at one year, 76% at two years, and 50% at five years, supporting the reliability of early and intermediate-term outcome estimates.

A total of 5,923 first HCTs performed from 2012 to 2024 were included in the analysis, consisting of 2,888 allogeneic and 3,035 autologous procedures. Information was originated from 19 public transplant centers participating in the RBTCH-TC. The median age at infusion was 31 years old (interquartile range = 15–48) for allogeneic recipients and 51 (interquartile range = 33–60) for autologous recipients. Among survivors, the median follow-up was 35.4 months (range 2–153) for allogeneic and 24.2 months (range 0–157) for autologous transplants.

Among allogeneic transplants, 46.2% were performed using human leukocyte antigen (HLA)-matched related donors, 28.1% with mismatched related donors, and 25.7% with unrelated donors. Acute leukemias accounted for most indications among allogeneic procedures (70.3%), whereas multiple myeloma was the most common diagnosis in the autologous group (51.2%). Additional demographic and transplant features are summarized in Table 2.

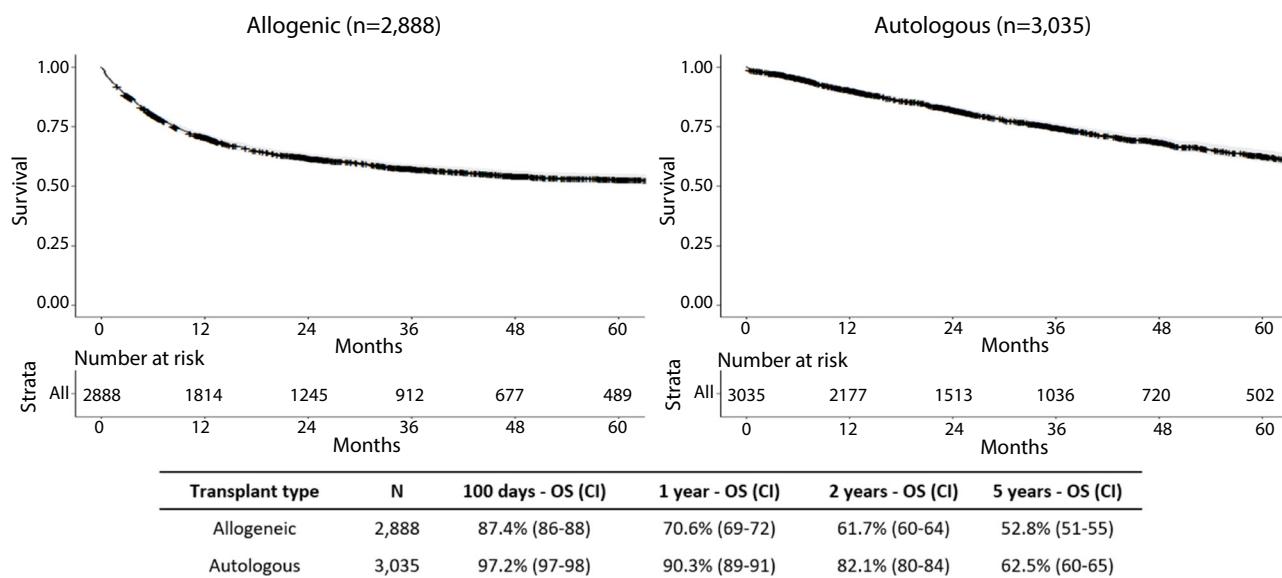
Table 2. Baseline characteristics of patients and transplant procedures in 19 public centers.

	Allogeneic	Autologous
Total	2,888	3,035
Patient age at HCT		
Median (IQR) (years old)	31 (15.48)	51 (33.60)
0–17	855 (29.6)	281 (9.3)
18–39	965 (33.4)	696 (22.9)
40–59	867 (30)	1253 (41.3)
60 or older	201 (7)	805 (26.5)
Gender		
Female	1226 (42.5)	1300 (42.8)
Male	1662 (57.5)	1735 (57.2)
Donor type		
Matched related donor	1335 (46.2)	-
Mismatch related donor	810 (28.1)	-
Unrelated donor	743 (25.7)	-
Donor gender		
Female	1042 (36.1)	-
Male	1666 (57.7)	-
Unknown	180 (6.2)	-
Median donor age (years old)	35.4 (1-78)	-
Diagnosis		
Aplastic anemia	7 (0.2)	0 (0)
Myeloproliferative neoplasm	69 (2.4)	0 (0)
Acute lymphoblastic leukemia	1018 (35.2)	3 (0.1)
Acute myeloid leukemia	1014 (35.1)	41 (1.4)
Chronic myeloid leukemia	253 (8.8)	0 (0)
Hodgkin lymphoma	11 (0.4)	575 (18.9)
Non-Hodgkin lymphoma	63 (2.2)	586 (19.3)
Multiple myeloma	1 (0)	1555 (51.2)
Other non-malignant diseases	9 (0.3)	0 (0)
Other leukemias	94 (3.3)	0 (0)
Other malignant diseases	2 (0.1)	275 (9.1)
Myelodysplastic syndrome	347 (12)	0 (0)
Product type		
Bone marrow	1476 (51.1)	24 (0.8)
PBSC	1390 (48.1)	3011 (99.2)
Cord blood	22 (0.8)	0 (0)
Performance status (%)		
90 or 100	2,456 (85)	2,401 (79.1)
< 90	399 (13.8)	583 (19.2)
Unknown	33 (1.1)	51 (1.7)
Last follow-up status		
Alive	1695 (58.7)	2264 (74.6)
Dead	1193 (41.3)	771 (25.4)
Median follow-up among survivors (months)	35.4 (2-153)	24.2 (0-157)

HCT: hematopoietic cell transplantation; IQR: interquartile range; PBSC: peripheral blood stem cells. Source: Elaborated by the authors.

Overall survival

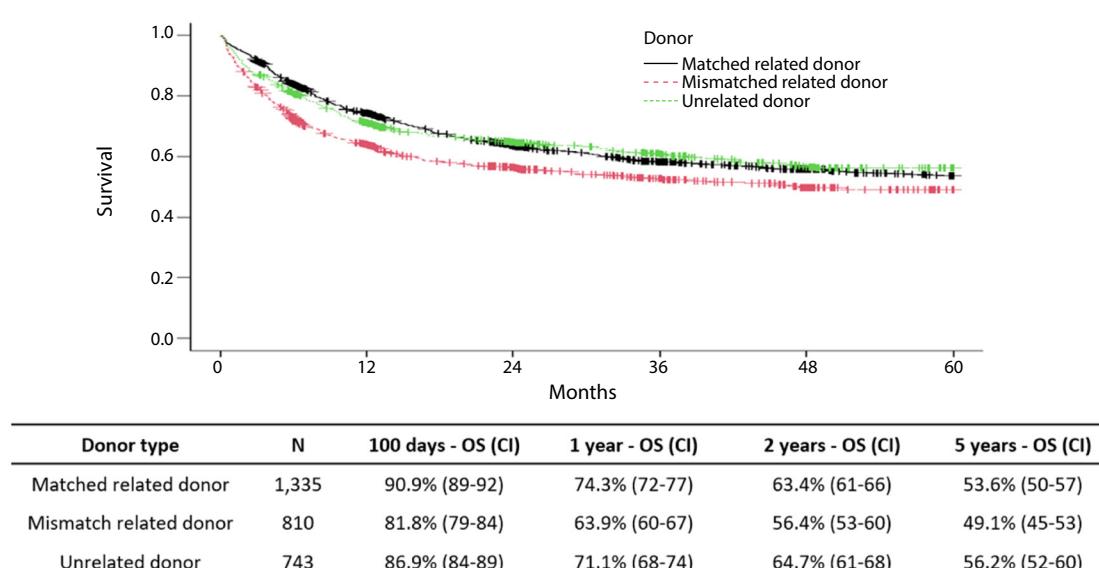
Overall survival was analyzed separately for autologous and allogeneic procedures. Among autologous recipients, estimated overall survival reached 97.2% at 100 days, 90.3% at one year, 82.1% at two years, and 62.5% at five years. For allogeneic HCT, overall survival rates were 87.4% at 100 days, 70.6% at one year, 61.7% at two years, and 52.8% at five years (Fig. 1).



OS: overall survival; CI: confidence interval. Source: Elaborated by the authors.

Figure 1. Overall survival curves for autologous and allogeneic hematopoietic cell transplantation.

Among allogeneic transplants, two-year overall survival differed according to donor category: 63.4% for matched related donors, 56.4% for mismatched related donors (including haploidentical), and 64.7% for unrelated donors ($p < 0.001$) (Fig. 2).

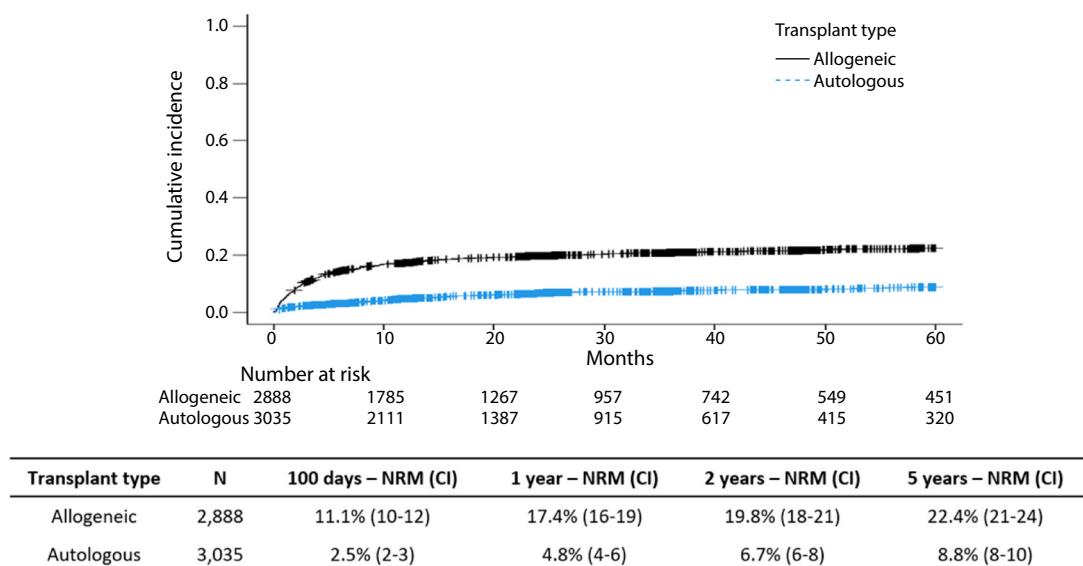


OS: overall survival; CI: confidence interval. Source: Elaborated by the authors.

Figure 2. Overall survival stratified by donor type in allogeneic hematopoietic cell transplantation.

Non-relapse mortality

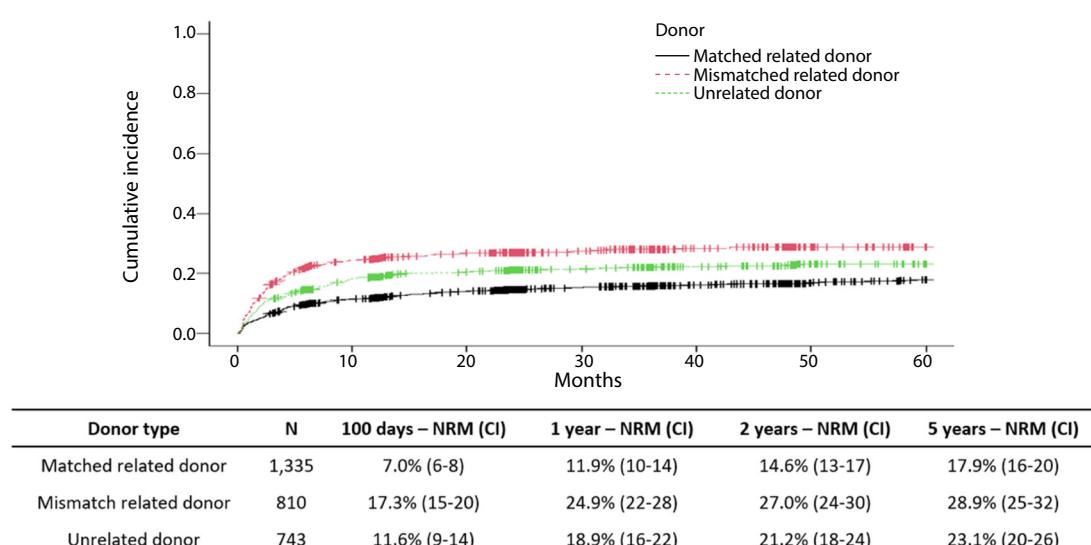
Among autologous recipients, NRM was 2.5% at 100 days to 4.8% at one year, 6.7% at two years, and 8.8% at five years. In the allogeneic cohort, the corresponding estimates were 11.1% at 100 days, 17.4% at one year, 19.8% at two years, and 22.4% at five years (Fig. 3).



CI: confidence interval. Source: Elaborated by the authors.

Figure 3. Cumulative incidence of non-relapse mortality in autologous and allogeneic hematopoietic cell transplantation.

At the two-year time point, NRM varied according to donor type: 14.6% for matched related donor, 27% for mismatched related donor, and 21.2% for unrelated donors ($p < 0.001$) (Fig. 4).

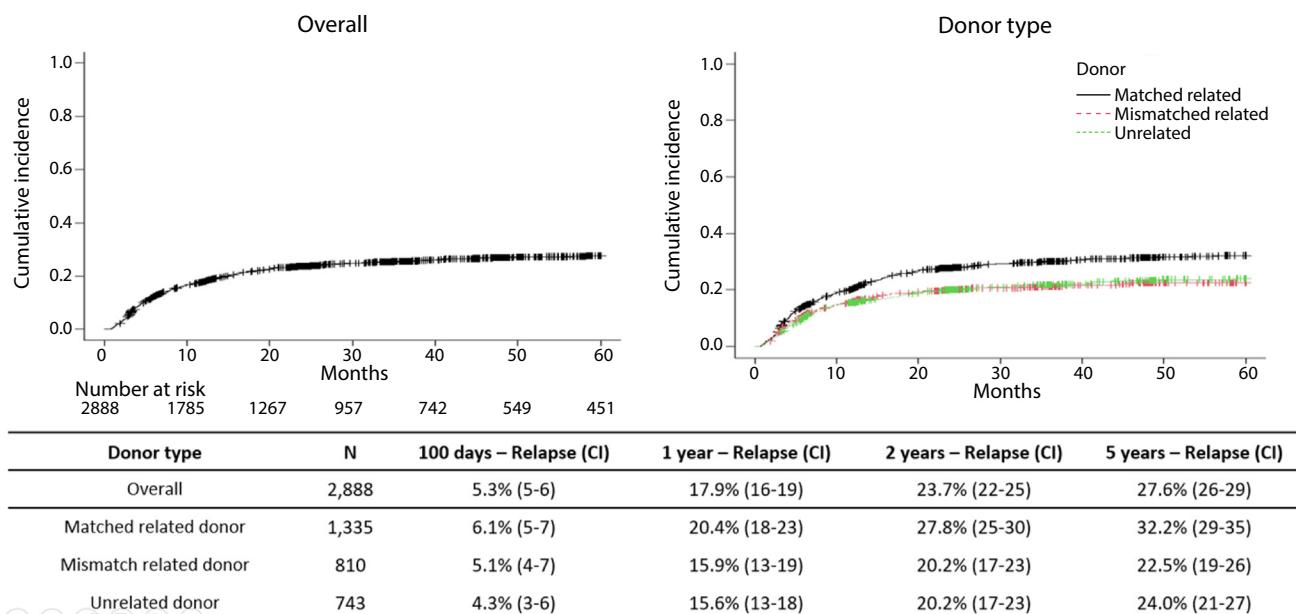


NRM: non-relapse mortality; CI: confidence interval. Source: Elaborated by the authors.

Figure 4. Cumulative incidence of non-relapse mortality stratified by donor type in allogeneic hematopoietic cell transplantation.

Relapse

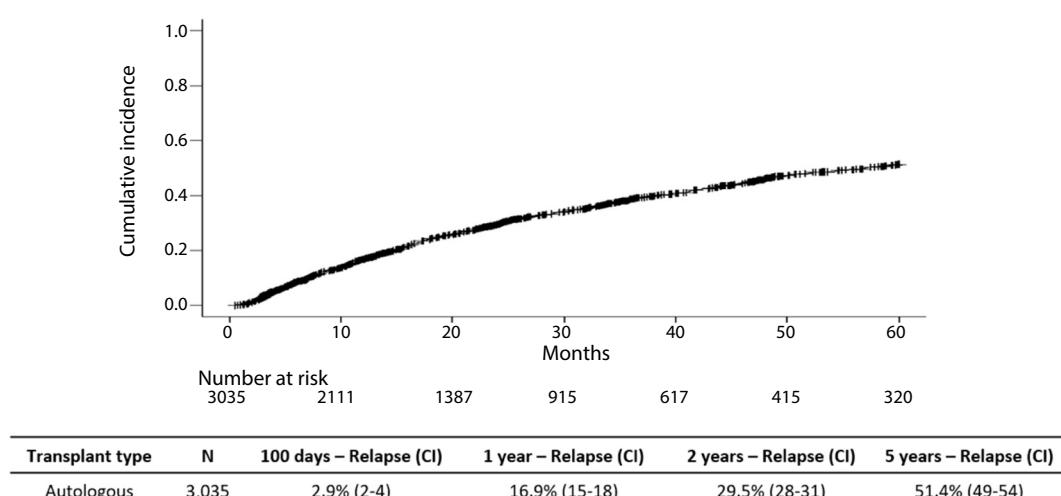
Among the 2,888 allogeneic transplants, the relapse incidence was 5.3% at 100 days, 17.9% at one year, 23.7% at two years, and 27.6% at five years. When we stratified by donor type, the cumulative incidence was 27.8% in matched related donors, and 20.2% in both mismatched related and unrelated donors ($p < 0.001$) (Fig. 5).



CI: confidence interval. Source: Elaborated by the authors.

Figure 5. Cumulative incidence of relapse in allogeneic hematopoietic cell transplantation: overall and stratified by donor type.

Among the 3,035 autologous procedures, the cumulative incidence of relapse was 2.9% at day 100, 16.9% at one year, 29.5% at two years, and 51.4% at five years (Fig. 6).

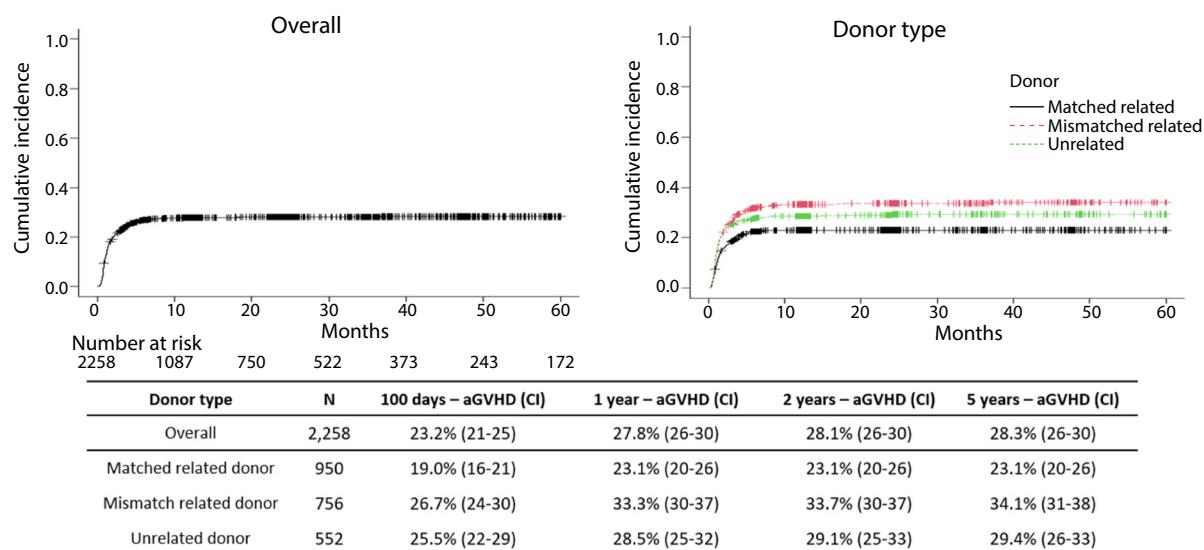


CI: confidence interval. Source: Elaborated by the authors.

Figure 6. Cumulative incidence of relapse in autologous hematopoietic cell transplantation.

Acute graft-versus-host disease

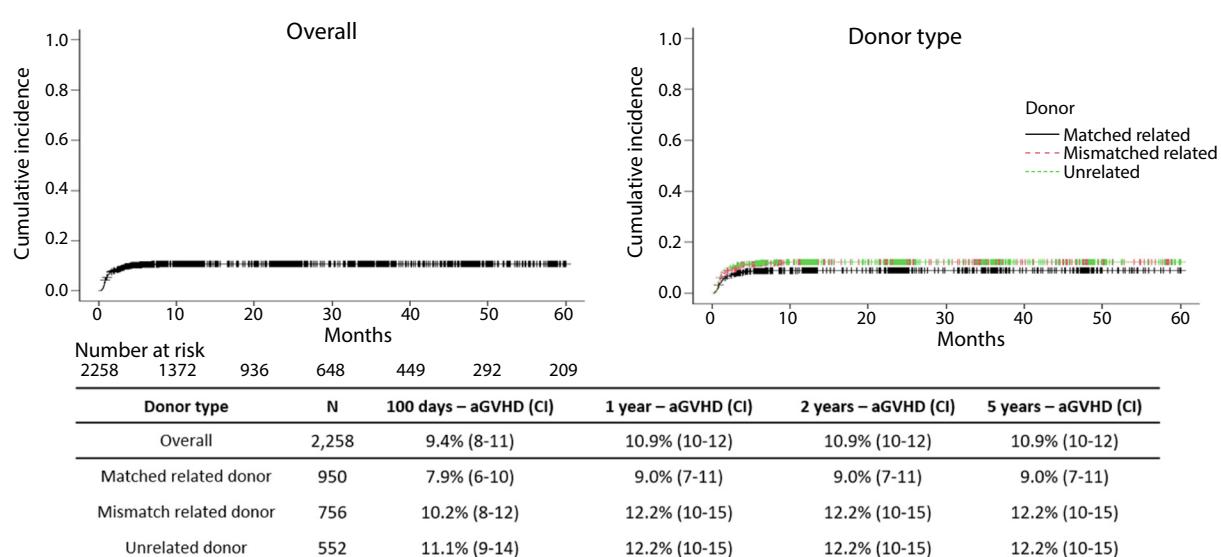
Among the 2,888 allogeneic transplants, 2,258 cases met the eligibility criteria for the aGVHD analysis. The incidence of grade II–IV aGVHD reached 23.2% at 100 days, increased to 27.8% at one year, and remained relatively stable at 28.1 and 28.3% at two and five years, respectively. At the two-year time point, donor type influenced risk: matched related donors had an incidence of 23.1%, compared with 33.7% for mismatched related donors and 29.1% for unrelated donors ($p < 0.001$) (Fig. 7).



aGVHD: acute graft-versus-host disease; CI: confidence interval. Source: Elaborated by the authors.

Figure 7. Cumulative incidence of acute graft-versus-host disease grade II–IV in allogeneic hematopoietic cell transplantation: overall and stratified by donor type.

For grade III–IV aGVHD, the cumulative incidence reached 9.4% at 100 days and rose to 10.9% at one year, remaining unchanged at both two and five years. At the two-year mark, incidence varied by donor type: 9% among matched related donors, 12.2% in mismatched related donors, and 12.2% in unrelated donors ($p = 0.056$) (Fig. 8).

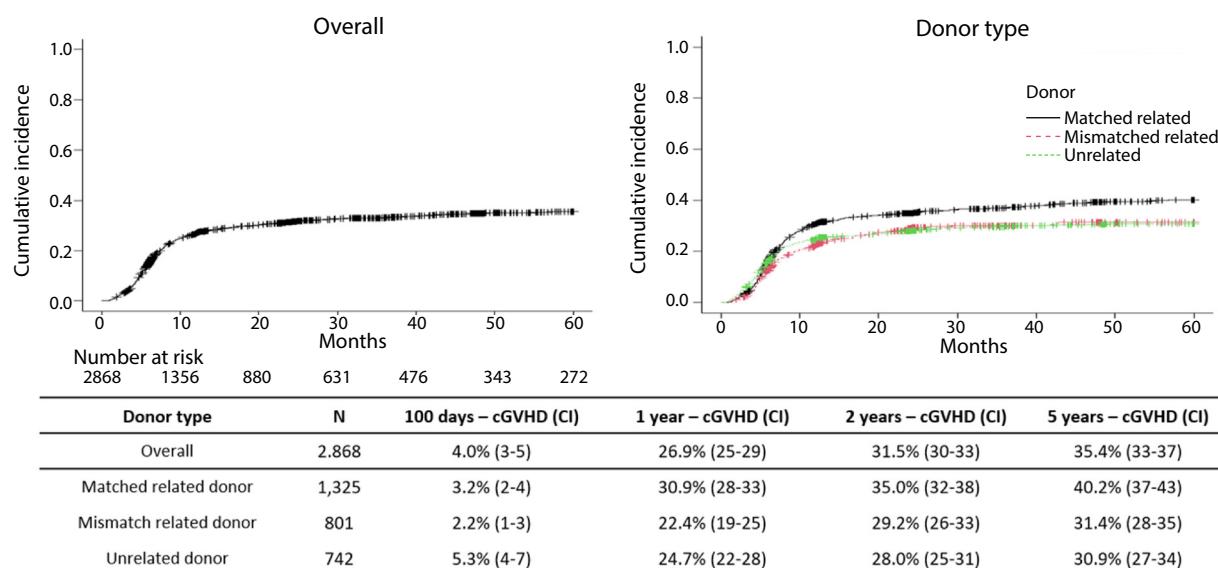


aGVHD: acute graft-versus-host disease; CI: confidence interval. Source: Elaborated by the authors.

Figure 8. Cumulative incidence of acute graft-versus-host disease grade III–IV in allogeneic hematopoietic cell transplantation: overall and stratified by donor type.

CHRONIC GRAFT-VERSUS-HOST DISEASE

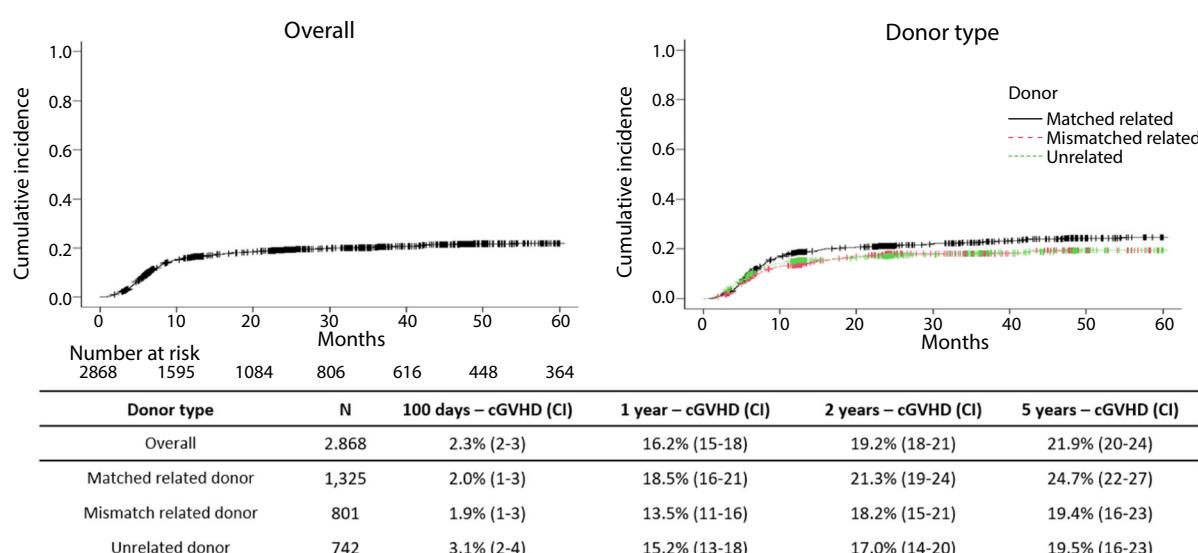
The evaluation of cGVHD comprised 2,868 allogeneic procedures, after excluding cases with absent or inconsistent cGVHD information. The cumulative incidence was 4% at 100 days, increasing to 26.9% at one year, 31.5% at two years, and reaching 35.4% at five years post-transplant. At two years, donor-specific incidence rates were 35% for matched related donors, 29.2% for mismatched related donors, and 28% for unrelated donors ($p = 0.0001$) (Fig. 9).



cGVHD: chronic graft-versus-host disease; CI: confidence interval. Source: Elaborated by the authors.

Figure 9. Cumulative incidence of chronic graft-versus-host disease in allogeneic hematopoietic cell transplantation: overall and stratified by donor type.

For moderate-to-severe cGVHD, the cumulative incidence reached 2.3% at 100 days, 16.2% after one year, 19.2% at two years, and 21.9% at five years. When stratified according to donor type, the two-year incidence was 21.3% for matched related donors, 18.2% for mismatched related donors, and 17% for unrelated donors ($p = 0.016$) (Fig. 10).



cGVHD: chronic graft-versus-host disease; CI: confidence interval. Source: Elaborated by the authors.

Figure 10. Cumulative incidence of moderate to severe chronic graft-versus-host disease in allogeneic hematopoietic cell transplantation: overall and stratified by donor type.

DISCUSSION

This analysis included data from 19 public transplant centers participating in the RBTCH-TC, encompassing nearly 6,000 first HCTs performed between 2012 and 2024. The study provides a comprehensive national overview of outcomes in the Brazil's public transplant network, offering meaningful insight into the performance, strengths, and challenges of centers operating under the Unified Health System (SUS). Despite resource constraints and structural heterogeneity, the outcomes observed in this cohort remain generally consistent with international benchmarks, reinforcing the overall quality of care delivered across public HCT programs^{10,11}.

NRM was notably higher among allogeneic transplant recipients, particularly during the first post-transplant year—a pattern aligned with international experience. Relapse, however, continued to represent the predominant cause of late mortality in both autologous and allogeneic settings. In autologous HCT, relapse rates increased progressively over time, exceeding 50% at five years, consistent with the natural history of diseases such as multiple myeloma and lymphoma.

The incidence of aGVHD was higher among recipients of mismatched related and unrelated donors, whereas cGVHD occurred more frequently in matched related donor transplants, highlighting the complex and multifactorial influence of donor type, conditioning intensity, and graft source on post-transplant outcomes. These findings reinforce the need for ongoing refinement of prophylaxis strategies and continued investment in long-term survivorship programs within the public network.

Despite the inherent challenges of a publicly funded health system, the overall alignment of national outcomes with global references underscores significant progress in Brazil's transplant capacity over the past decade. Sustained improvement in data quality, integration between centers, and adoption of standardized care pathways remain essential to further reducing early mortality and enhancing long-term results.

The retrospective design of the study and variability in follow-up completeness across centers represent acknowledged limitations. Nonetheless, the breadth and consistency of data derived from 19 public centers provide a representative and reliable national perspective on transplant outcomes, supporting both benchmarking and health-policy planning.

In recent years, targeted initiatives within the RBTCH-TC have led to meaningful improvements in follow-up completeness and data reliability. Compared with previous national reports, the median follow-up increased substantially for both autologous and allogeneic HCTs. These developments reflect strengthened data-management practices and growing institutional engagement, contributing to more accurate outcome assessments and supporting continuous quality improvement across Brazil's public transplant network.

CONCLUSION

This study provides the first national overview of HCT outcomes in the Brazil's public healthcare network, based on data from 19 participating centers. Survival rates, relapse, and NRM were consistent with international benchmarks, demonstrating that high-quality transplant care can be achieved within a publicly funded system.

The findings underscore the importance of maintaining comprehensive follow-up and continuous data monitoring across centers to identify opportunities for improvement and support evidence-based decision-making. Strengthening collaborative data initiatives such as the RBTCH-TC is essential to guide quality improvement efforts, promote transparency, and enhance patient outcomes throughout Brazil's public transplant network.

CONFLICT OF INTEREST

Nothing to declare.

DATA AVAILABILITY STATEMENT

Data sharing is not applicable.

AUTHORS' CONTRIBUTIONS

Substantive scientific and intellectual contributions to the study: Simione AJ, Silva CC, Hamerschlak N, Vigorito AC, Neves HRA, Seber A, Bonfim CMS, Colturato VAR, Nabhan SK, Rocha VG, Barros GMN, Lerner D, Daudt LE, Fernandes JF, Colella MP, Silverio A, Soares RDA, Teixeira GM, Zecchin VG, Hallack Neto AE, Schaffel R, Calixto RF, Franco SCR, Veloso GDC, Funke VAM, Duarte FB. **Conception and design:** Duarte, FB. **Analysis and interpretation of data:** Simione AJ, Silva CC, Duarte FB. **Technical procedures:** Simione AJ, Silva CC, Duarte FB. **Statistics analysis:** Simione AJ. **Manuscript writing:** Simione AJ. **Final approval:** Simione AJ, Silva CC, Hamerschlak N, Vigorito AC, Neves HRA, Seber A, Bonfim CMS, Colturato VAR, Nabhan SK, Rocha VG, Barros GMN, Lerner D, Daudt LE, Fernandes JF, Colella MP, Silverio A, Soares RDA, Teixeira GM, Zecchin VG, Hallack Neto AE, Schaffel R, Calixto RF, Franco SCR, Veloso GDC, Funke VAM, Duarte FB.

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DECLARATION OF USE OF ARTIFICIAL INTELLIGENCE TOOLS

The authors declare that artificial intelligence tools were used solely to assist with language editing, grammar refinement, and clarify of expression during manuscript preparation. No artificial intelligence tools were used for data analysis, interpretation of results, or generation of scientific content. The authors retain full responsibility for the content of this manuscript.

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Supplementary Table 1. List of participating public transplant centers.

Center name
Centro de Pesquisas Oncológicas Dr. Alfredo Daura Jorge (CEPON)
CTMO-HCFMUSP
Fundação Pio XII - Hospital de Câncer de Barretos
Hospital Amaral Carvalho
Hospital da Criança de Brasília José Alencar
Hospital de Clínicas - UFPB
Hospital de Clínicas de Porto Alegre
Hospital Pequeno Príncipe
Hospital Universitário Clementino Fraga Filho, Univ. Fed. RJ
Hospital Universitário da Universidade Federal de Juiz de Fora
Hospital Universitário Walter Cantídio/UFC
Instituto da Criança - Hospital das Clínicas da Faculdade de Medicina Universidade de São Paulo
Instituto de Oncologia Pediátrica - GRAACC
Instituto Nacional de Câncer
Natal Hospital Center
Real Hospital Português
Santa Casa de Montes Claros
UFMG Hospital das Clínicas Serviço de Transplante de Medula Óssea
UNICAMP – HEMOCENTRO