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Insights of pediatric bone marrow transplantation in Brazil - A registry-based study (2018–2024)

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ABSTRACT

Background: Hematopoietic cell transplantation (HCT) is established as a curative strategy for several severe pediatric hematological and immunological disorders. However, despite Brazil's extensive public transplant infrastructure, national data on pediatric HCT trends remain insufficiently characterized. **Objective:** To analyze temporal trends, regional distribution, and modality characteristics of pediatric HCT in Brazil from 2018 to 2024, including the impact of the COVID-19 pandemic. **Methods:** This retrospective descriptive study used data from the Brazilian Transplant Registry (RBT/ABTO). Descriptive statistics were used and transplant modalities were compared across pre-pandemic (2018–2019), pandemic (2020–2022), and post-pandemic (2023–2024) periods using Pearson's chi-square test. **Results:** A total of 3,592 pediatric BMT procedures were reported. Allogeneic transplantation predominated throughout the study period (68.3%–87.5%), while the proportion of autologous procedures increased significantly during the pandemic ($p < 0.001$). São Paulo concentrated more than half of all procedures, indicating substantial regional concentration. The lowest annual volume occurred in 2024 ($n = 377$), potentially reflecting incomplete or revised registry data. **Conclusion:** The data showed temporal variations, pandemic-associated changes in transplantation modalities, and persistent regional disparities. These findings highlight the complexity of pediatric transplant care in Brazil and the need for standardized data collection and policies to reduce access inequities.

Keywords: Hematopoietic cell transplantation; Allogeneic transplantation; Autologous transplantation; COVID-19 pandemic.

INTRODUCTION AND EVOLVING FRAMEWORK OF SURVIVORSHIP CARE

Hematopoietic cell transplantation (HCT), also known as bone marrow transplantation (BMT), consists of replacing diseased bone marrow with healthy cells from bone marrow, peripheral blood, or umbilical cord blood, and is indicated for a wide range of hematological, immunological, and genetic diseases.[1]

Despite advances in conditioning regimens, donor selection, and graft-versus-host disease (GVHD) prophylaxis — with long-term survival exceeding 80% in certain immunodeficiency conditions — pediatric HCT remains associated with substantial acute and late complications, including severe infections, GVHD, organ toxicities, osteonecrosis, growth disturbances, renal dysfunction, and long-term endocrine, pulmonary, cardiovascular, and neurological sequelae requiring ongoing multidisciplinary follow-up.[2–9]

The COVID-19 pandemic added further complexity by introducing increased infectious risk, logistical constraints, and delays in transplant activity at national and international levels.[10–12] In Brazil, these challenges were compounded by structural and organizational constraints within the public health system, potentially exacerbating regional disparities in access to HCT and affecting continuity of care.[13–15]

Although epidemiological studies on HCT exist, few have analyzed the impact of the COVID-19 pandemic specifically on pediatric BMT procedures in Brazil. A detailed understanding of temporal trends, regional disparities in access, and the pandemic's specific impact on transplant modality distribution is essential to inform healthcare policy and expand equitable access to this life-saving intervention. The present study addresses this gap using publicly available national registry data from 2018 to 2024.

MATERIALS AND METHODS

This retrospective, descriptive, quantitative study used publicly available secondary data from the Brazilian Transplant Registry (RBT) of the Brazilian Association of Organ Transplantation (ABTO).[16] The study was conducted in accordance with the 1964 Helsinki Declaration (Bull World Health Organ 2001;79:373–374), and the protocol was approved by the Ethics Committee of the Hospital de Clínicas de Porto Alegre (Project no.: 2025-0316; CAAE no.: 90758525.8.0000.5327).

All pediatric BMT procedures performed in patients aged 0 to 17 years between January 2018 and December 2024 were included. Variables analyzed were: geographic region and state, transplant modality (autologous or allogeneic), absolute number of procedures (N), rate per million pediatric population (pmp), number of solid organ transplants (SOT) for contextual reference, pediatric donors, and deaths on the pediatric solid organ transplant waiting list (included as a contextual indicator, given the absence of a national HCT-specific waiting list registry).

Data were organized in spreadsheets and subjected to descriptive statistical analysis, with calculation of absolute and relative frequencies, means, and measures of dispersion when applicable. BMT rates per million pediatric population were calculated using population estimates from the Children and Adolescents Observatory (IBGE). To evaluate differences in transplant modality distribution across pandemic periods, Pearson's chi-square test was performed comparing pre-pandemic (2018–2019), pandemic (2020–2022), and post-pandemic (2023–2024) periods. Statistical significance was set at $p < 0.05$.

RESULTS

Between 2018 and 2024, a total of 3,592 pediatric BMT procedures were performed in Brazil. Annual volumes ranged from 413 transplants in 2018 to 619 in both 2021 and 2023 (Table 1). A decline was recorded in 2020 (n = 477), coinciding with the onset of the COVID-19 pandemic. From 2021 onwards, a sustained recovery was observed, with volumes remaining above 540 per year through 2023. In 2024, a total of 377 procedures were recorded, reflecting partial-year or adjusted data as reported by ABTO.

Both solid organ transplants (SOT) and BMT procedures evolved differently over time. In some years, BMT remained stable or grew while SOT declined, reinforcing the operational and clinical independence between these two types of transplantation. The BMT rate per million pediatric population (pmp) followed a similar trend, rising from 6.8 pmp in 2018 to 8.6 pmp in 2019, declining to 7.7 pmp in 2020, and reaching its highest values of 9.8 pmp in 2021 and 2023 (Table 1).

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Table 1. Pediatric transplants in Brazil (2018–2024).

Year	Ped. Pop. (million)	SOT	Pediatric Donors	Deaths on List	BMT (N)	Autologous BMT n (%)	Allogeneic BMT n (%)	Bone Marrow pmp
2018	61.17	600	229	80	413	94 (22.8%)	319 (77.2%)	6.8
2019	61.87	584	235	64	534	124 (23.2%)	410 (76.8%)	8.6
2020	61.87	486	214	56	477	151 (31.7%)	326 (68.3%)	7.7
2021	62.85	583	200	82	619	194 (31.3%)	425 (68.7%)	9.8
2022	62.85	553	188	66	553	162 (29.3%)	391 (70.7%)	8.8
2023	62.85	574	274	79	619	172 (27.8%)	447 (72.2%)	9.8
2024	62.85	555	223	93	377	47 (12.5%)	330 (87.5%)	8.7

SOT = Solid organ transplants (liver, kidney, heart); BMT = Bone marrow transplant; pmp = per million pediatric population. Pediatric population expressed in millions of inhabitants. Deaths on list correspond to annual pediatric waiting list deaths for solid organ transplants. Source: Elaborated by the authors using data from RBT/ABTO and Children and Adolescents Observatory – IBGE.

Transplant modality: autologous versus allogeneic

Allogeneic transplants predominated throughout the study period, representing more than 68% of annual procedures in all years (Table 1). During the pandemic period (2020–2022), a statistically significant proportional increase in autologous transplants was observed, reaching 31.7% in 2020 and 31.3% in 2021. In the post-pandemic period, allogeneic procedures returned to higher proportions, reaching 87.5% of all BMTs in 2024.

Pearson's chi-square test confirmed a statistically significant association between the pandemic period and transplant modality distribution ($\chi^2 = 31.64$; df = 2; $p < 0.001$), as shown in Table 2.

Table 2. Comparison of autologous and allogeneic transplant proportions across pandemic periods in the pediatric group in Brazil.

Bone marrow transplant procedures	Pre-pandemic (2018-2019) (N=947) n(%)	Pandemic (2020-2022) (N=1,649) n(%)	Post-pandemic (2023-2024) (N=996) n(%)	*p-value*
Autologous	218 (23.02)	507 (30.75)	219 (21.99)	1.35×10^{-7}
Allogeneic	729 (76.98)	1,142 (69.25)	777 (78.01)	

Data are presented in absolute numbers and percentages. The comparison of transplant modality proportions between pre-pandemic (2018–2019), pandemic (2020–2022), and post-pandemic (2023–2024) periods was performed using Pearson's chi-square test ($p < 0.05$). Source: Elaborated by the authors using data from RBT/ABTO.

Access to transplantation and waiting list mortality

Despite the increase in absolute HCT procedure volumes, deaths on the pediatric solid organ transplant (SOT) waiting list — used here as an indirect, contextual indicator of strain on the broader pediatric transplant system, since no equivalent national waiting-list metric exists for HCT — remained high throughout the study period, with a growing trend in recent years (Table 1). The lowest number of SOT waiting list deaths was recorded in 2020 (n = 56), while the highest was observed in 2024 (n = 93).

Regional distribution

The Southeast Region concentrated the majority of BMT procedures, consistently accounting for more than half of annual procedures — both allogeneic and autologous. São Paulo stood out as the principal transplant hub in all evaluated years, representing between 46% and 65% of allogeneic procedures. The South Region ranked second, with a notable contribution from Paraná. The Northeast Region showed a smaller yet relatively stable share, primarily driven by Pernambuco and, more recently, Rio Grande do Norte. The Midwest Region, particularly Goiás and the Distrito Federal, demonstrated a rise in allogeneic transplant activity from 2021 onward. The North Region recorded a complete absence of BMT activity in every year of the study period (2018–2024) (Table 3 and 4) — the only Brazilian region with zero registered procedures throughout the entire series.

Table 3. Distribution of bone marrow transplants by federal unit in Brazil, pediatric group (2018–2020).

Region/State	2018 allo BMT (N=319)	2018 auto BMT (N=94)	2019 allo BMT (N=410)	2019 auto BMT (N=124)	2020 allo BMT (N=326)	2020 auto BMT (N=151)
Southeast region						
São Paulo	147 (46.1%)	55 (58.5%)	248 (60.5%)	70 (56.5%)	196 (60.1%)	75 (49.7%)
Rio de Janeiro	13 (4.1%)	8 (8.5%)	19 (4.6%)	9 (7.3%)	21 (6.5%)	13 (8.6%)
Minas Gerais	18 (5.6%)	10 (10.6%)	8 (2.0%)	17 (13.7%)	14 (4.3%)	4 (2.7%)
South region						
Paraná	100 (31.4%)	5 (5.3%)	83 (20.2%)	10 (8.1%)	52 (16.0%)	13 (8.6%)
Santa Catarina	2 (0.6%)	0	1 (0.2%)	0	5 (1.5%)	0
Rio Grande do Sul	21 (6.6%)	8 (8.5%)	16 (3.9%)	12 (9.7%)	11 (3.4%)	18 (11.9%)
Northeast region						
Ceará	1 (0.3%)	0	0	1 (0.8%)	0	1 (0.7%)
Pernambuco	12 (3.8%)	4 (4.3%)	20 (4.9%)	5 (4.0%)	19 (5.8%)	4 (2.7%)
Rio Grande do Norte	0	0	3 (0.7%)	1 (0.8%)	0	2 (1.3%)
Bahia	0	0	1 (0.2%)	1 (0.8%)	4 (1.2%)	1 (0.7%)
Midwest region						
Goiás	3 (0.9%)	1 (1.1%)	2 (0.5%)	4 (3.2%)	0	0
Distrito Federal	4 (1.3%)	3 (3.2%)	1 (0.2%)	3 (2.4%)	1 (0.3%)	3 (2.0%)

Source: Elaborated by the authors using data from RBT/ABTO.

Table 4. Distribution of bone marrow transplants by federal unit in Brazil, pediatric group (2021–2024).

Region/State	2021 allo BMT (N=425)	2021 auto BMT (N=194)	2022 allo BMT (N=391)	2022 auto BMT (N=162)	2023 allo BMT (N=447)	2023 auto BMT (N=172)	2024 allo BMT (N=330)	2024 auto BMT (N=47)
Southeast region								
São Paulo	235 (55.3%)	100 (51.6%)	255 (65.2%)	94 (58.0%)	241 (53.9%)	116 (67.4%)	157 (47.6%)	30 (63.8%)
Rio de Janeiro	24 (5.7%)	9 (4.6%)	15 (3.8%)	13 (8.0%)	23 (5.1%)	9 (5.2%)	9 (2.7%)	2 (4.3%)
Minas Gerais	20 (4.7%)	10 (5.2%)	11 (2.8%)	14 (8.6%)	5 (1.1%)	19 (11.1%)	0	13 (27.7%)
South region								
Paraná	104 (24.5%)	14 (7.2%)	61 (15.6%)	7 (4.3%)	83 (18.6%)	3 (1.7%)	94 (28.5%)	0
Santa Catarina	1 (0.2%)	5 (2.6%)	0	0	3 (0.7%)	0	0	0
Rio Grande do Sul	25 (5.9%)	8 (4.1%)	17 (4.4%)	1 (0.6%)	21 (4.7%)	10 (5.8%)	14 (4.2%)	2 (4.3%)
Northeast region								
Ceará	1 (0.2%)	1 (0.5%)	3 (0.8%)	1 (0.6%)	0	0	1 (0.3%)	0
Pernambuco	18 (4.2%)	3 (1.6%)	0	0	19 (4.3%)	7 (4.1%)	20 (6.1%)	0
Rio Grande do Norte	12 (2.8%)	1 (0.5%)	0	0	15 (3.4%)	1 (0.6%)	5 (1.5%)	0
Bahia	6 (1.4%)	1 (0.5%)	3 (0.8%)	4 (2.5%)	4 (0.9%)	0	0	10 (21.3%)
Midwest region								
Goiás	0	0	0	0	0	0	0	0
Distrito Federal	10 (2.4%)	13 (6.7%)	9 (2.3%)	2 (1.2%)	18 (4.0%)	17 (9.9%)	17 (5.2%)	0

alloBMT = allogeneic bone marrow transplant; autoBMT = autologous bone marrow transplant. Values expressed as absolute numbers (n) and percentages (%). States with no recorded procedures throughout the study period (Mato Grosso do Sul, Mato Grosso, Espírito Santo, Paraíba, Maranhão, Piauí, Sergipe, Alagoas, and all states of the North region) are not shown. Source: Elaborated by the authors using data from RBT/ABTO.

DISCUSSION

Pediatric BMT is essential for the treatment of several serious hematological, immunological, and genetic diseases in childhood. The present results demonstrate that, from 2018 to 2024, pediatric BMT in Brazil showed a general upward trend, despite occasional fluctuations.

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The decline observed in 2020 — both in absolute BMT numbers and in age-adjusted rates — is consistent with the global impact of the COVID-19 pandemic on health systems. The pandemic led to structural changes in transplant center protocols, including systematic screening of patients, donors, and healthcare professionals; postponement of non-urgent procedures; and reorganization of hospital workflows to mitigate viral transmission risk.[10,11,14,15]

These measures, while essential for protecting immunocompromised patients, contributed to temporary reductions in transplant activity and accumulation of unmet demand. Such changes may have altered patient selection, timing of transplantation, and donor availability, with potential downstream effects on early post-transplant risks, non-relapse mortality, and GVHD incidence.[4,5,17]

The high proportion of allogeneic transplants (exceeding 70% in most years) is consistent with international registry data and reflects the clinical profile of pediatric HCT indications, which differ markedly from adult practice.[2,11]

The proportional increase in autologous transplants during the pandemic period (2020–2022) may reflect operational and clinical factors: autologous procedures do not depend on external donors, involve lower immunological complexity, and carry lower infectious risk — characteristics that confer procedural advantage in resource-constrained environments.[7,11,14]

The return to a pronounced predominance of allogeneic transplants in the post-pandemic period supports this interpretation, suggesting normalization of donor chains and care flows.

Despite increasing BMT procedure volumes, deaths on the solid organ transplant waiting list, the only waiting list metric available in the national registry, and not a direct measure of HCT waitlist mortality, remained high throughout the study period. While this metric cannot be directly attributed to HCT, its persistence alongside growing BMT volumes is consistent with a broader imbalance between transplant demand and available care capacity, and points to structural inequality in access to transplantation in Brazil.[18,19] This conceptual limitation should be considered when interpreting waitlist-related findings in the present study.

The marked geographic concentration of procedures in the Southeast Region — especially São Paulo — and the complete absence of transplant activity in the North Region throughout the entire 2018–2024 period highlight deeply rooted structural barriers. Based on regional population estimates (IBGE, Censo 2022 and subsequent projections), the North Region's pediatric population is estimated at approximately at 5–6 million children and adolescents; this figure is an indirect approximation derived by applying national age-bracket proportions to regional population totals, rather than a value drawn directly from the Observatório da Criança e Adolescente (IBGE) as used for the national pediatric population in Table 1, and should be interpreted with corresponding caution. Across seven consecutive years, this population had no locally available HCT center and depended entirely on referral to other regions for a potentially curative, time-sensitive procedure. This geographic barrier — combined with the unequal distribution of specialized transplant centers, limited availability of multidisciplinary teams in peripheral regions, and insufficient hospital infrastructure — is consistent with patterns documented in Latin American and national analyses. These include the unequal distribution of specialized transplant centers, limited availability of multidisciplinary teams in peripheral regions, and insufficient hospital infrastructure, patterns also documented in Latin American and national analyses.[11, 17–19]

This study has limitations inherent to the use of secondary administrative data, including the absence of clinical outcomes (survival, engraftment rates, GVHD incidence), incomplete state-level records for several years, and the lack of indication-specific data. The 2024 data may represent a partial-year or adjusted figure and should be interpreted with caution. Future studies incorporating clinical outcome data and indication-level analysis would substantially strengthen the evidence base for policy decisions.

CONCLUSION

Pediatric Hematopoietic Cell Transplantation (HCT) in Brazil demonstrated resilience and progressive recovery following the COVID-19 pandemic, with procedure volumes and rates per million population surpassing pre-pandemic levels from 2021 onwards. However, persistently high solid organ transplant waiting list mortality (used as a contextual indicator) and marked regional concentration — especially in the Southeast and South, contrasted with the complete absence of activity in the North — underscore the urgent need to expand transplant capacity, strengthen donor recruitment, and address structural inequities in access to pediatric HCT across Brazilian regions.

ETHICAL APPROVAL

The study was conducted in accordance with the 1964 Helsinki Declaration (Bull World Health Organ 2001;79:373–374), and the protocol was approved by the Ethics Committee of the Hospital de Clínicas de Porto Alegre (Project no.: 2025-0316; CAAE no.: 90758525.8.0000.5327).

CONFLICTS OF INTEREST

The authors declare no conflict of interest.

DATA AVAILABILITY STATEMENT

The data used in this study are publicly available through the Brazilian Transplant Registry (RBT) published by the Brazilian Association of Organ Transplantation (ABTO) at: https://site.abto.org.br/publicacoes/rbt

AUTHOR'S CONTRIBUTIONS

Conceptualization: Silva SK, Paim JSX, Seki JM; **Investigation:** Silva SK; **Methodology:** Silva SK, Seki JM; **Formal Analysis:** Silva SK; **Data Curation:** Silva SK; **Writing – Original Draft:** Silva SK; Stüker JB, Law LGM, Bortolon LP, Coronel SNCC, Gonçalves MS, Pinto SCR, Souza IRS, Longhi NCS. **Writing – Review & Editing:** Paim JSX, Stüker JB, Seki JM, Freitas EC. **Supervision:** Paim JSX, Freitas EC. **Final Approval:** Silva SK.

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DECLARATION OF USE OF ARTIFICIAL INTELLIGENCE TOOLS

During the preparation of this work, the authors used ChatGPT to assist with English writing. After using this tool/service, the authors reviewed and edited the content as needed and take full responsibility for the content of the published article.

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