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SHORT REPORT

Incidence and risk factors of graft failure in allogeneic hematopoietic stem cell transplantation for mucopolysaccharidosis in a nationwide pediatric cohort. A study on behalf of the Francophone Society of Bone Marrow Transplantation and Cellular Therapy

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Abstract

Context: Mucopolysaccharidosis (MPS) requires urgent treatment to prevent neurological damage. While gene therapy holds promise for effectively treating these diseases with minimal toxicity, access remains limited for most patients. Consequently, advancing allogeneic hematopoietic stem cell transplantation (HSCT) for young children is crucial. Since the 2010s, cord blood (CB) transplants with reduced-toxicity conditioning (RTC) have become the standard of care.

Patients and methods: Recent reports in France indicate a significant incidence of graft failures (GF), prompting a large-scale retrospective study from the French-speaking bone marrow transplantation society's registry, to understand GF risks, guide clinicians in selecting transplant platforms, and describe outcomes of second HSCT in young patients.

Results: This report analyses 93 children who underwent HSCT for MPS between 2000 and 2020. The GF rate was notably high (22.6% at day 100), primarily associated with the donor's HLA compatibility and the recipient's age. Well-matched CB and RTC were not found to be risk factors for GF. This study also details the procedures for second and third transplants in patients who rejected their first HSCT.

Conclusion: In the era of RTC, CB remains a viable and expedient option for MPS transplantation.

KEYWORDS
cord blood, graft failure, GVHD, HSCT, mucopolysaccharidosis, outcomes, reduced-toxicity conditioning

1 | CONTEXT

Mucopolysaccharidoses (MPS) are rare lysosomal storage disorders characterized by severe neurological impairment, especially in MPS-I, where cognitive decline occurs in the absence of effective treatment. Intravenous enzyme replacement therapy (ERT) has been a standard treatment treatment for MPS-I since 2003 [1]. However, this synthetic enzyme does not cross the blood-brain barrier, leaving neurological damage untreated [2].

Gene therapy, long-studied, is expected to yield fewer complications than hematopoietic stem cell transplantation (HSCT) [3], but it remains inaccessible to most patients.

Thus, HSCT performed as early as possible, remains a first-line treatment to prevent central nervous system damage [4]. Numerous successful HSCTs for MPS have been reported worldwide, achieving normal enzyme levels [5] and significantly improving long-term survival [6, 7].

In urgent therapeutic contexts where neurocognitive sequelae must be limited, cord blood (CB) is frequently used due to its rapid availability. The significant benefits of CB for HSCT in MPS were first demonstrated in 2004 [8] and led to its establishment as the preferred source for achieving normal enzyme levels by 2013 [9]. Two large studies in 2013 and 2015 reported a complete donor chimerism rate following CB transplantation in about 90% of patients [5, 10]. Since 2010, CB has been considered superior to other graft sources, including matched sibling donors (MSD), particularly in cases involving heterozygous donors, leading to a lower final enzyme level. This advantage is attributed to the reduced time from diagnosis to transplantation and the achievement of stable and optimal enzyme blood levels [9].

Meanwhile, advancements in transplant medicine have optimized conditioning regimens. Traditional myeloablative conditioning regimens combining high doses of busulfan and cyclophosphamide (Bu-Cy), predominantly used before 2010, have been largely replaced by reduced toxicity regimens (RTC), primarily based on busulfan and fludarabine (Bu-Flu), demonstrating similar efficacy with reduced toxicity.

However, following the adoption of these new protocols for MPS patients, many cases of graft failure (GF) have been reported in recent years to our national pediatric HSCT board, highlighting the need to define the modalities for second transplants. This study, conducted at the request of pediatric hematologists of the Francophone Society of Bone Marrow Transplantation and Cell Therapy (SFGM-TC), aims to evaluate the evolution of transplant strategies in MPS patients from 2000 to 2020, focusing on GF incidence and risk factors.

Data were collected from the European bone marrow transplantation registry. Informed consent was obtained from all parents, and each participating transplant center agreed to be involved in the study.

TABLE 1 Patients and transplants' characteristics.

Variable	Category	*N*/median	%/IQR
Sex	M	36	39%
	F	57	61%
Age	months	11	7–17
Type of MPS	I	85	92%
	II	3	3%
	VI	1	1%
	VII	2	2%
	Unknown	2	2%
Weight	kg	11	11.2–12.6
Height	cm	80	75.7–85
Number of transplants	1	78	84%
	2	14	15%
	3	1	1%
Age at transplant	months	18	13–24
Stem cell graft	CB	44	48%
	BM	46	49%
	PBSC	2	2%
	Unknown	1	1%
Donor (all graft sources)	MSD	14	15%
	MUD	39	42%
	MMUD	27	39%
	UD not otherwise specified	13	14%
ABO matching	Matched	31	33%
	Minor incompatibility	27	29%
	Major incompatibility	25	27%
	Unknown	10	11%
CMV status	D+/R+	10	11%
	D+/R-	19	20%
	D-/R+	10	11%
	D-/R-	51	55%
	Unknown	3	3%
Conditioning regimen	Bu-Cy	46	49%
	Bu-Flu	37	40%
	Other	8	9%
	Unknown	2	2%
2000–2009 **2010–2020**	BuCy BuFlu		87% 70%
Graft richness	**TNC (10^7/kg)**		
	BM/PBSC	58.5	32.4–100
	CB	8.5	5.1–13.2
	CD34 (10^6/kg)		
	BM/PBSC	9.3	6.7–17.2
	CB	0.3	0.2–0.5
	CD3 (10^7/kg)		
	BM/PBSC	43.2	31–69
	CB	15.8	13.4–20.6
Busulfan pharmacokinetics (AUC, mg × h/L)	Missing data on 44 patients	85.2	75.2–97.8
Serotherapy	Yes	76	82%
	No	15	16%
	Unknown	2	2%
ATG dose	mg/kg	10	7.5–10
GVHD prophylaxis	Calcineurin inhibitor only	20	21%
	Calcineurin inhibitor + MTX	18	20%
	Calcineurin inhibitor + MMF	28	30%
	Calcineurin inhibitor + corticosteroids	25	27%
	Other	2	2%

Abbreviations: ATG: antithymoglobulins; BM: bone marrow; Bu: busulfan (myeloablative dose); CB: cord blood; Cy: cyclophosphamide; Flu: fludarabine; MMF: mycophenolate mofetil; MMUD: mismatched unrelated donor; MPS: mucopolysaccharidosis; MSD: matched sibling donor; MTX: methotrexate; MUD: matched unrelated donor; PBSC: peripheral blood stem cell; UD: unrelated donor (missing data for HLA matching).

Inclusion criteria encompassed all children with MPS type I, II, VI, or VII who received HSCT between 2000 and 2020 at a center affiliated with the SFGM-TC Society.

GF was classified as primary when chimerism was above 50% recipient origin at day 30, and secondary when chimerism dropped below 50% donor origin after initial neutrophil engraftment (>500/mm^3) and initial complete donor (>99%) or mixed (50%–99%) chimerism.

All statistical tests were two-sided, and a p-value < 0.05 was considered statistically significant.

Statistical analyses were performed using R-Project 4.0, with two-sided tests and a significant level set at $p < 0.05$. Details on statistical methods and Busulfan pharmacokinetics assessments are provided in the Supporting Information Methods.

2 | RESULTS

2.1 | Patient and transplantation characteristics

Between June 2000 and July 2020, 93 patients underwent their first HSCT for MPS across 13 centers (Table 1). Notably, 43% were transplanted before 2010. The median age at diagnosis was 11 months (range 7.0–17.0), and the median age at first HSCT was 18 months

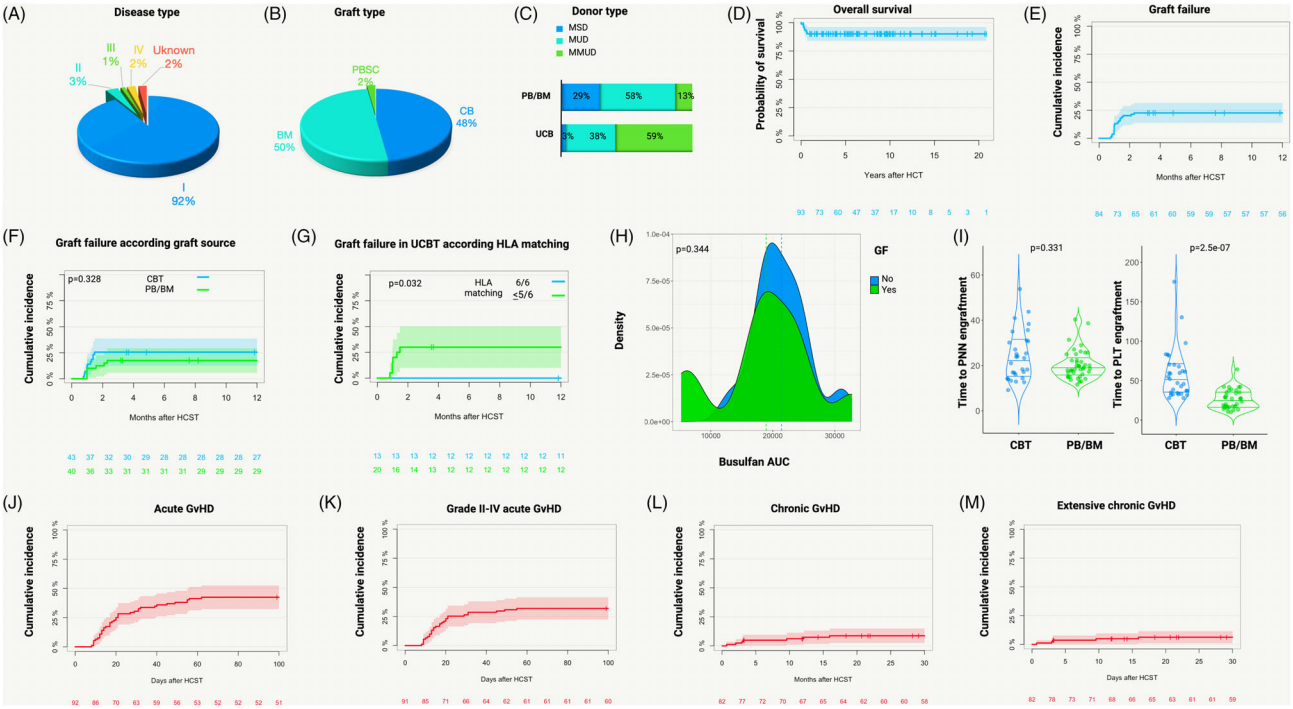

FIGURE 1 (A) Eighty-five patients of the cohort had type I-mucopolysaccharidosis, three type II, one type VI, and two type VII. (B) Allogeneic transplantations were performed with cord blood (CB) for 48% of patients ($n = 44$), with bone marrow (BM) for 50% of patients ($n = 46$), and with peripheral blood stem cell (PBSC), for 2% of patients ($n = 2$) (1 patient with missing data). (C) For patients who received bone marrow (BM) or peripheral blood stem cell (PBSC), 29% received a graft from a matched sibling donor (MSD), 58% from a matched unrelated donor (MUD, defined as HLA 10/10 matching), and 13% from a mismatched unrelated donor (MMUD, defined as < 10/10 HLA matching). For cord blood grafts, 3% were from an MSD, 38% were matched (MUD, defined as 6/6 HLA matching, with HLA-A and B serotyping and HLA-DR allelic typing), while 59% were mismatched (3 to 5/6 HLA matching). (D) The probability of survival for overall survival (OS) was calculated using Kaplan-Meier estimates. OS was defined as the time from first transplant to death for any cause. OS at 1 year was 90.1% (CI 84.1–96.4). (E) Cumulative incidences of graft failure (GF) were calculated in a competing risk setting, where death was considered the competing event. The cumulative incidence of GF was 11.9% on day 30 and 22.6% on day 100 with no further events after day 100. (F) The cumulative incidence of GF according to stem cell source did not differ between CB and BM/PBSC (respectively 25% vs. 18% at 3 months, $p = 0.328$). (G) Cumulative incidence of GF for CB transplantations, according to HLA matching. No event occurred in the 6/6 HLA-matched CB transplantations, whereas the cumulative incidence of GF for ≤ 5/6 HLA-matched CB reached 30% at 3 months (in a competing risk setting). (H) Density plot showing the area under the curve of Busulfan (in μM.min) comparing patients who experienced GF vs patients who had a sustained engraftment after the first transplantation. (I) Illustration of time to neutrophil (left) and platelet (right) reconstitution according to stem cell source. Engraftment was defined for granulocytes as the first day of reaching neutrophils > 0.5 G/L for at least three consecutive days and for platelets as the first day with platelets > 50 G/L for at least seven consecutive days without transfusion. (J–M) Cumulative incidence of graft versus host disease (GVHD) after the first transplant was assessed in a competitive risk setting, with death at +100 day for acute and at any time for chronic GVHD considered as a competing event, and with patients alive without GVHD censored at the last follow-up. (J) Overall acute GVHD CI was 42.4% at day 100. (K) Grades II–IV acute GVHD CI at D100 was estimated at 31.9%. (L) Chronic GvHD CI at 1 year was 6 .1%. (M) Chronic extensive GVHD CI at 1 year was 4.9%.

(range 13.0–24.0). CB was the graft source for 49% of patients, while bone marrow (BM) or peripheral blood stem cells (PBSCs) were used for 50%. A slight majority (57%) of patients receiving BM/PBSC were transplanted from matched unrelated donors (MUDs), while 59% of CB transplants were performed with mismatched unrelated units (defined as 3–5 out of six human leukocyte antigen-HLA-compatibility) (Figure 1A–C). Serotherapy was administered to 78 patients (84%), consisting of antithymoglobulins (ATG) for all but one patient who received alemtuzumab. ATG was given 3 or 4 days prior to transplantation for BM and PBSCs, starting earlier (around day 9) for most cord blood transplantations with available timing data (Table 1).

All patients received a myeloablative regimen: Bu-Cy in 49% (mostly between 2000 to 2009) and Bu-Flu in 40% (predominantly after 2010).

Pharmacokinetics data for Busulfan were available for 49 patients, with a median area under the curve (AUC) of 85.2 mg × h/L (range 21.4–134.6).

2.2 | Outcomes

With a median follow-up of 6 years (range 2.0–9.0), overall survival at 1 year was 90.1% (cumulative incidence [CI] 84.1–96.4) with no further events occurring after 6 months (Figure 1D). Two patients were lost to follow-up, and nine died from various causes including respiratory disease ($n = 4$), multiorgan failure ($n = 2$), false route ($n = 1$), veno-occlusive disease ($n = 1$), and graft-versus-host disease (GVHD, $n = 1$). Univariate

analysis revealed no factors, including graft source, donor type, or conditioning regimen, significantly influencing survival (Table S1).

Nineteen of 91 patients experienced GF, with CI rates of 11.9% at day 30 and 22.6% at day 100, with no further events beyond day 100 (Figure 1E). Sixteen patients (84%) had primary GF, while three (16%) experienced secondary GF, exclusively among those receiving BM/PBSCs. The median time to GF was 42 days (range 31.0–64.0) post-HSCT.

Univariate analysis identified recipient age (hazard ratio [HR] 1.04 [95%CI 1.01–1.07], $p = 0.022$) and HLA matching as significant risk factors for GF, with mismatched donors showing a heightened risk (HR 5.8 [95%CI 1.98–17], $p = 0.001$, Table S2). The graft source was not associated with rejection (HR = 0.63 for BM/PBSC vs. CB, $p = 0.32$). Among CB recipients, HLA matching at 6/6 correlated with a significantly lower CI of GF (Figure 1F,G). Notably, neither transplant period (before or after 2010) nor conditioning regimens (Bu-Cy vs. RTC), significantly impacted GF rates. Busulfan pharmacokinetics assessment showed no differences regarding GF incidence (Figure 1H). Platelet engraftment was significantly delayed in CB transplants compared to BM or PBSC, though median times for neutrophil engraftment were similar across graft sources (Figure 1I).

The overall 100-day CI of acute GVHD was 42.4% ([95%CI 32.1–52.3], Figure 1J), with a grades II–IV acute GVHD CI estimated at 31.9% ([95%CI 22.5–41.6], Figure 1K). At 1 year, the CI of chronic GvHD was 6.1%, ([95%CI 2.3–12.8], Figure 1L) and for chronic extensive GVHD, it was 4.9% ([95%CI 1.6–11.2], Figure 1M).

2.3 | Outcomes after further transplant strategies

Fourteen patients (15%) underwent two HSCTs, and one patient received three transplants (1%) (Table S3). Four patients who experienced GF after the first HSCT did not proceed to second transplantation; of these, one passed away, while three were alive at the last follow-up.

The median age at the second transplant was 23 months (range 21.0–29.0). Neutrophil engraftment occurred at a median of 22 days (range 17.5–27.75), while median times for platelet engraftment above 20 and 50 G/L thresholds were 19 (16.0–40.0) and 23 days (16.75–37.75) post-HSCT.

After this second HSCT, five of the 15 patients experienced another GF, with four cases being primary and one secondary. Two patients died after their second HSCT, one from multiorgan failure and the other from a false route.

One patient successfully underwent a third transplant following a myeloablative conditioning regimen, achieving complete donor chimerism. Despite developing early grade III acute GVHD, this patient was alive at the last follow-up, 4 years post-transplant.

Among patients who achieved sustained engraftment, the mean level of last known chimerism was 96% for BM/PBSC (range 50%–100%, $n = 41$ patients with available data), and 98% for CB (range 89%–100%, $n = 37$ patients with available data), with no significant difference ($p = 0.30$).

3 | KEY POINTS AND CONCLUSION

While gene therapy remains inaccessible to most patients, HSCT continues to be a critical intervention. This study emphasizes the need for close monitoring of chimerism until at least day 100 and adapting transplantation strategies to improve outcomes and effectively manage GF. The GF incidence in this study was higher than reported in historical studies (22% vs. <5%–16%) [5, 10], potentially due to differing GF definitions (chimerism <10% of donor origin for Boelens et al.). Patient age and HLA compatibility were the only significant factors associated with GF, while graft source and type of conditioning regimen were not identified as risk factors.

Based on these findings, we recommend that clinicians prioritize the most compatible grafts, especially for older patients. This may involve selecting an MSD who does not carry the mutation or a matched CB unit. Historically, and for almost half of our cohort, HLA typing of cord blood was evaluated at low resolution for loci A and B loci, and at high resolution for DRB1. Post-2012, centers have progressively implemented allelic typing of 8 alleles (A, B, C, and DRB1), in line with international recommendations [11], highlighting the survival advantages, and reduced toxic mortality associated with 8/8 high-resolution compatible CB versus 7/8 and 6/8 [12, 13].

Given the prognostic significance of HLA compatibility on rejection, and since 6/6 CBs (high resolution) can be compatible from 6/10 to 10/10, we advocate for choosing the most compatible CB possible based on allelic typing of at least eight or even 10 alleles (+ DPB1). Additionally, we recommend monitoring Busulfan AUC, as its efficacy in ensuring engraftment has been previously demonstrated [14], despite its limited impact in our cohort.

In the era of reduced toxicity conditioning, CB remains a viable and expedient option for MPS transplantation.

AUTHOR CONTRIBUTIONS

Laura Danhardt, Cecile Pochon, and Simona Pagliuca wrote the manuscript. Simona Pagliuca and Arnaud Wiedemann performed statistical analyses. Simona Pagliuca performed data visualization. Gerard Michel, Jean-Hugues Dalle, Fanny Rialland, Cécile Renard, Charlotte Jubert, Johan Maertens, Anne Sirvent, Nimrod Buchbinder, Christine Devalck, Bénédicte Brichard, Catherine Paillard, and Martin Castelle were in charge of MPS transplanted patients in their respective centers and Stephanie Nguyen, President of the Scientific Committee of SFGMTC society, responsible for the registry, helped to edit and proofread the manuscript. David Combarel and Angelo Paci provided data on busulfan pharmacokinetics and helped edit and proofread the manuscript.

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CONFLICT OF INTEREST STATEMENT

This research was conducted in the absence of any commercial or financial relationships that could be construed as a potential conflict of interest. The authors declare no conflict of interest.

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The authors received no specific funding for this work.

DATA AVAILABILITY STATEMENT

The data that support the findings of this study are available from the corresponding author [Cecile Pochon] upon reasonable request.

ETHICS STATEMENT

The authors have confirmed ethical approval statement is not needed for this submission.

PATIENT CONSENT STATEMENT

The scientific committee of SFGM-TC (Société Francophone de Greffe de Moelle et de Thérapie Cellulaire) approved the study and data were collected through the European bone marrow transplantation (EBMT) registry. The transplantation centers verified recorded data and were asked to provide missing information. All parents of the patients provided informed written consent for the EBMT/SFGM-TC registry, and each participating transplant center agreed to be involved in the study.

CLINICAL TRIAL REGISTRATION

The authors have confirmed clinical trial registration is not needed for this submission.

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SUPPORTING INFORMATION

Additional supporting information can be found online in the Supporting Information section at the end of this article.

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