

JACIE Cumulative Outcomes Report

2025 Annual Report - Rolling Cohort Analysis

HSCT Program Quality Indicators

2026-01-23

i AI-Generated Report with Rolling Cohort Methodology

Executive Summary

Reporting Period and Methodology

Reporting Period: Calendar Year 2025 (January 1 - December 31, 2025)

Methodology: Cumulative Rolling Cohort Analysis following JACIE Italian guidelines

Cumulative Rolling Cohort Methodology

This report uses **cumulative rolling cohort analysis** as specified by JACIE Italy. For each quarter and timepoint, we calculate outcomes for ALL patients who reached that milestone during the cumulative period from Q1 through that quarter.

Key principle: Results should be **cumulative and monotonically increasing** - the percentage at 100 days should be ≥ 30 days, and 365 days should be ≥ 100 days, since we're looking at the same patients plus those who reach later milestones.

Example for Q4 2025:

- **30-day outcome:** All patients whose 30-day milestone fell between **Jan 1 - Nov 30, 2025** (transplanted Sep 1, 2024 - Oct 31, 2025)
- **100-day outcome:** All patients whose 100-day milestone fell between **Jan 1 - Sep 30, 2025** (transplanted Jul 1, 2024 - Sep 30, 2025)
- **365-day outcome:** All patients whose 365-day milestone fell between **Jan 1 (2024) - Dec 31, 2025** (transplanted Jan 1, 2024 - Dec 31, 2025)

This provides mature outcome data since patients have had sufficient follow-up time.

Key Findings Summary

Overall Mortality (All Patients) - Cumulative Rolling Cohort

Table 1: Table 1: Overall Cumulative Mortality - Rolling Cohort Analysis

Timepoint	Cohort Size	Deaths	Mortality Rate
30 days	30	0	0% (0/30)
100 days	29	1	3.4% (1/29)
365 days	20	7	35% (7/20)

Understanding Cohort Sizes

Note that cohort sizes **differ** across timepoints because each represents a different observation period:

- **30-day cohort:** Patients whose 30-day milestone occurred Jan 1 - Nov 30, 2025 (N = 30)
- **100-day cohort:** Patients whose 100-day milestone occurred Jan 1 - Sep 30, 2025 (N = 29)
- **365-day cohort:** Patients whose 365-day milestone occurred Jan 1, 2024 - Dec 31, 2025 (N = 20)

The mortality percentages are cumulative and should generally increase at later timepoints (30d 100d 365d) as cumulative mortality accrues over time.

Mortality by Cause: TRM, NRM, and RRM

Table 2: Table 2: Cumulative Mortality Breakdown by Cause (Overall = TRM + NRM + RRM)

Timepoint	Overall Mortality	TRM	NRM	RRM
30 days	0% (0/30)	0% (0/30)	0% (0/30)	0% (0/30)
100 days	3.4% (1/29)	0% (0/29)	3.4% (1/29)	0% (0/29)
365 days	35% (7/20)	15% (3/20)	10% (2/20)	10% (2/20)

Understanding Mortality Categories

TRM (Treatment-Related Mortality) includes deaths directly related to the transplant procedure and immediate complications.

NRM (Non-Relapse Mortality) includes deaths from:

- Infection
- GVHD complications
- Organ toxicity
- Other transplant-related complications (not classified as TRM)

RRM (Relapse-Related Mortality) includes deaths from disease progression or relapse.

At 365 days: **TRM** = 15%, **NRM** = 10%, and **RRM** = 10%

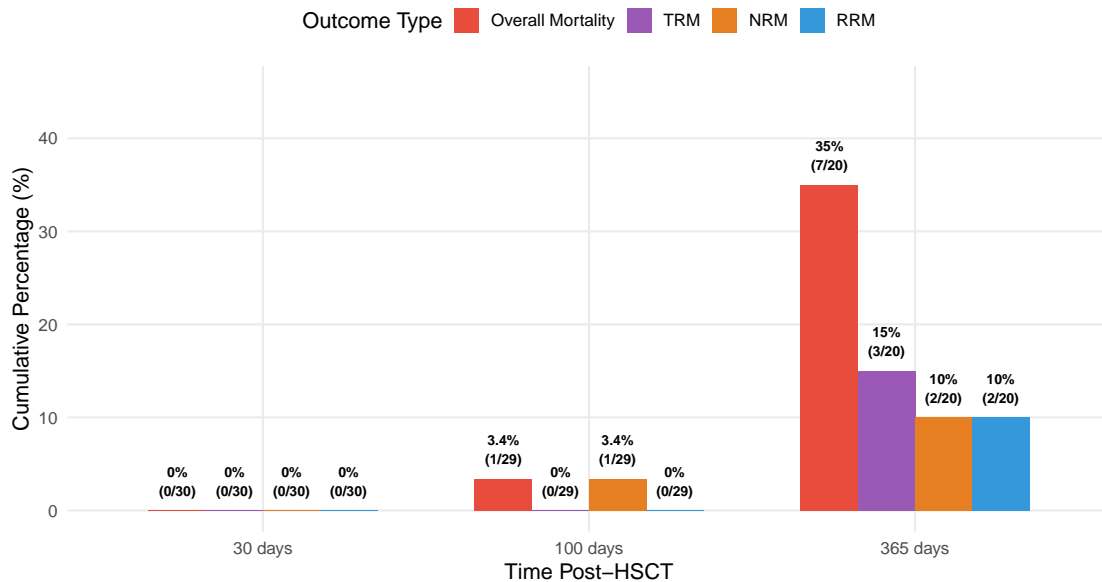
Detailed Results

1. Overall Mortality Analysis

1.1 Mortality at Key Timepoints by Cause

Figure 1: Cumulative Mortality Outcomes by Timepoint

Cumulative Rolling Cohort Analysis – 2025 (TRM, NRM, RRM)



1.2 Mortality by Disease Status at Transplant

Table 3: Table 3: Cumulative Mortality by Disease Status at HSCT

Group	Timepoint	Cohort Size	Deaths	Mortality Rate
Complete Remission				
Complete Remission	30 days	23	0	0% (0/23)
Complete Remission	100 days	16	0	0% (0/16)
Complete Remission	365 days	11	2	18.2% (2/11)
Not in Complete Remission				
Not in Complete Remission	30 days	7	0	0% (0/7)
Not in Complete Remission	100 days	13	1	7.7% (1/13)
Not in Complete Remission	365 days	9	5	55.6% (5/9)

Disease Status Impact

Complete Remission patients: 365-day mortality = 18.2%

Not in Complete Remission: 365-day mortality = 55.6%

Achieving complete remission before transplant is associated with better outcomes.

2. Non-Relapse Mortality (NRM)

2.1 NRM by Timepoint

Table 4: Table 4: Cumulative Non-Relapse Mortality (NRM)

Timepoint	Cohort Size	NRM Events	NRM Rate
30 days	30	0	0% (0/30)
100 days	29	1	3.4% (1/29)
365 days	20	2	10% (2/20)

NRM Analysis

Definition: NRM represents deaths from transplant-related complications, NOT disease progression.

- **30-day NRM:** 0% - Early transplant complications
- **100-day NRM:** 3.4% - Standard early NRM window
- **365-day NRM:** 10% - Cumulative NRM including late complications

NRM accounts for **29%** of all deaths at 365 days.

2.2 NRM by Disease Status

Table 5: Table 5: Non-Relapse Mortality by Disease Status

Group	Timepoint	Cohort Size	NRM Events	NRM Rate
Overall				
Overall	30 days	30	0	0% (0/30)
Overall	100 days	29	1	3.4% (1/29)
Overall	365 days	20	2	10% (2/20)
Complete Remission				
Complete Remission	30 days	23	0	0% (0/23)
Complete Remission	100 days	16	0	0% (0/16)
Complete Remission	365 days	11	1	9.1% (1/11)
Not in Complete Remission				
Not in Complete Remission	30 days	7	0	0% (0/7)
Not in Complete Remission	100 days	13	1	7.7% (1/13)
Not in Complete Remission	365 days	9	1	11.1% (1/9)

2.3 NRM Causes Breakdown

Table 6: Table 6: Breakdown of NRM by Specific Cause (365-day cohort)

Cause of NRM	N Events	Percentage
Other	2	2 (100%)

2.4 TRM (Treatment-Related Mortality)

2.4.1 TRM by Timepoint

Table 7: Table 6A: Cumulative Treatment-Related Mortality (TRM)

Timepoint	Cohort Size	TRM Events	TRM Rate
30 days	30	0	0% (0/30)
100 days	29	0	0% (0/29)
365 days	20	3	15% (3/20)

TRM Analysis

Definition: TRM represents deaths specifically from treatment-related complications (distinct from NRM and relapse).

- **30-day TRM:** 0%
- **100-day TRM:** 0%
- **365-day TRM:** 15%

TRM accounts for **43%** of all deaths at 365 days.

2.4.2 TRM by Disease Status

Table 8: Table 6B: Treatment-Related Mortality by Disease Status

Group	Timepoint	Cohort Size	TRM Events	TRM Rate
Overall				
Overall	30 days	30	0	0% (0/30)
Overall	100 days	29	0	0% (0/29)
Overall	365 days	20	3	15% (3/20)
Complete Remission				
Complete Remission	30 days	23	0	0% (0/23)
Complete Remission	100 days	16	0	0% (0/16)
Complete Remission	365 days	11	1	9.1% (1/11)
Not in Complete Remission				
Not in Complete Remission	30 days	7	0	0% (0/7)
Not in Complete Remission	100 days	13	0	0% (0/13)
Not in Complete Remission	365 days	9	2	22.2% (2/9)

3. Relapse-Related Mortality (RRM)

Table 9: Table 7: Cumulative Relapse-Related Mortality (RRM)

Timepoint	Cohort Size	RRM Deaths	RRM Rate
30 days	30	0	0% (0/30)
100 days	29	0	0% (0/29)
365 days	20	2	10% (2/20)

3.1 RRM by Disease Status

Table 10: Table 8: Relapse-Related Mortality (RRM) by Disease Status

Group	Timepoint	Cohort Size	RRM Deaths	RRM Rate
Overall				
Overall	30 days	30	0	0% (0/30)
Overall	100 days	29	0	0% (0/29)
Overall	365 days	20	2	10% (2/20)
Complete Remission				
Complete Remission	30 days	23	0	0% (0/23)
Complete Remission	100 days	16	0	0% (0/16)
Complete Remission	365 days	11	0	0% (0/11)
Not in Complete Remission				
Not in Complete Remission	30 days	7	0	0% (0/7)
Not in Complete Remission	100 days	13	0	0% (0/13)
Not in Complete Remission	365 days	9	2	22.2% (2/9)

RRM Summary

- **365-day RRM:** 10%
- RRM accounts for **29%** of all deaths at 365 days
- Not in CR patients show higher RRM (22.2%) vs CR patients (0%)

Late Relapse Deaths (After 365 Days)

Important Note: The 365-day rolling cohort includes **** 20 patients**** whose 365-day milestone fell during the reporting period. Of these:

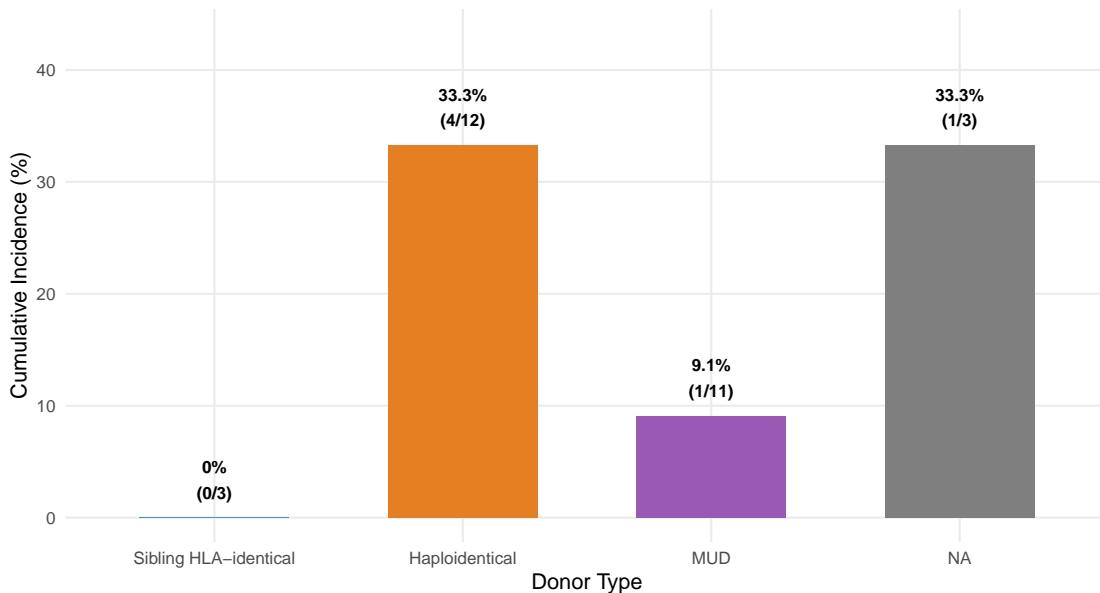
- **** 2 patients**** died from relapse **within 365 days** post-HSCT (included in 365-day RRM rate above)
- **** 1 additional patient(s)**** died from relapse **after 365 days** post-HSCT:
 - Patient GF60: Death at day **428** (63 days after 365-day milestone)

These late deaths are correctly EXCLUDED from the 365-day mortality analysis as they occurred beyond the 365-day timepoint. They would be captured in longer-term follow-up analyses (e.g., 2-year or 3-year mortality).

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Figure 2: Acute GVHD Grade II–IV by Donor Type

Cumulative incidence at 100 days post-HSCT (Cumulative Rolling Cohort)



4.2 Chronic GVHD (365 days)

Table 11: Table 10: Chronic GVHD Cumulative Incidence (365 days)

Severity	Cohort Size	Events	Cumulative Incidence
Any	20	2	10% (2/20)
Moderate-Severe	20	1	5% (1/20)

5. Comprehensive Outcomes by Donor Type

Table 12: Table 11: Comprehensive Outcomes by Donor Type

Donor Type	365-Day Mortality	NRM at 365 Days	Acute GVHD II-IV
Sibling HLA-identical	0% (0/2)	0% (0/2)	0% (0/3)
Haploidentical	50% (6/12)	16.7% (2/12)	33.3% (4/12)
MUD	20% (1/5)	0% (0/5)	9.1% (1/11)
MMUD	0% (0/1)	0% (0/1)	33.3% (1/3)

6. Mortality Outcomes by Donor Type

6.1 Overall Mortality by Donor Type (All Timepoints)

Table 13: Table 13: Overall Mortality by Donor Type Across All Timepoints

Donor Type	Timepoint	Cohort Size	Deaths	Mortality Rate
Sibling HLA-identical				
Sibling HLA-identical	30 days	3	0	0% (0/3)
Sibling HLA-identical	100 days	3	0	0% (0/3)
Sibling HLA-identical	365 days	2	0	0% (0/2)
Haploidentical				
Haploidentical	30 days	9	0	0% (0/9)
Haploidentical	100 days	12	1	8.3% (1/12)
Haploidentical	365 days	12	6	50% (6/12)
MUD				
MUD	30 days	12	0	0% (0/12)
MUD	100 days	11	0	0% (0/11)
MUD	365 days	5	1	20% (1/5)
MMUD	30 days	6	0	0% (0/6)
MMUD	100 days	3	0	0% (0/3)
MMUD	365 days	1	0	0% (0/1)

6.2 NRM by Donor Type (All Timepoints)

Table 14: Table 14: Non-Relapse Mortality by Donor Type Across All Timepoints

Donor Type	Timepoint	Cohort Size	NRM Events	NRM Rate
Sibling HLA-identical				
Sibling HLA-identical	30 days	3	0	0% (0/3)
Sibling HLA-identical	100 days	3	0	0% (0/3)
Sibling HLA-identical	365 days	2	0	0% (0/2)
Haploidentical				
Haploidentical	30 days	9	0	0% (0/9)
Haploidentical	100 days	12	1	8.3% (1/12)
Haploidentical	365 days	12	2	16.7% (2/12)
MUD				
MUD	30 days	12	0	0% (0/12)
MUD	100 days	11	0	0% (0/11)
MUD	365 days	5	0	0% (0/5)
MMUD	30 days	6	0	0% (0/6)
MMUD	100 days	3	0	0% (0/3)
MMUD	365 days	1	0	0% (0/1)

6.3 Relapse Mortality by Donor Type (All Timepoints)

Table 15: Table 15: Relapse Mortality by Donor Type Across All Timepoints

Donor Type	Timepoint	Cohort Size	Relapse Deaths	Relapse Rate
Sibling HLA-identical				
Sibling HLA-identical	30 days	3	0	0% (0/3)
Sibling HLA-identical	100 days	3	0	0% (0/3)
Sibling HLA-identical	365 days	2	0	0% (0/2)
Haploidentical				
Haploidentical	30 days	9	0	0% (0/9)
Haploidentical	100 days	12	0	0% (0/12)
Haploidentical	365 days	12	1	8.3% (1/12)
MUD				
MUD	30 days	12	0	0% (0/12)
MUD	100 days	11	0	0% (0/11)
MUD	365 days	5	1	20% (1/5)
MMUD	30 days	6	0	0% (0/6)
MMUD	100 days	3	0	0% (0/3)
MMUD	365 days	1	0	0% (0/1)

6.4 TRM by Donor Type (All Timepoints)

Table 16: Table 15A: Treatment-Related Mortality by Donor Type Across All Timepoints

Donor Type	Timepoint	Cohort Size	TRM Events	TRM Rate
Sibling HLA-identical				
Sibling HLA-identical	30 days	3	0	0% (0/3)
Sibling HLA-identical	100 days	3	0	0% (0/3)
Sibling HLA-identical	365 days	2	0	0% (0/2)
Haploidentical				
Haploidentical	30 days	9	0	0% (0/9)
Haploidentical	100 days	12	0	0% (0/12)
Haploidentical	365 days	12	3	25% (3/12)
MUD				
MUD	30 days	12	0	0% (0/12)
MUD	100 days	11	0	0% (0/11)
MUD	365 days	5	0	0% (0/5)
MMUD	30 days	6	0	0% (0/6)
MMUD	100 days	3	0	0% (0/3)
MMUD	365 days	1	0	0% (0/1)

7. Combined Stratification: Donor Type × Disease Status

7.1 Overall Mortality by Donor Type and CR Status (All Timepoints)

Table 17: Table 16: Overall Mortality by Donor Type and Disease Status (30, 100, 365 days)

CR Status	Donor Type	Timepoint	N	Deaths	Mortality Rate (n/N)
Complete Remission	Sibling HLA-identical	30 days	1	0	0% (0/1)
Complete Remission	Sibling HLA-identical	100 days	1	0	0% (0/1)
Complete Remission	Sibling HLA-identical	365 days	2	0	0% (0/2)
Complete Remission	Haploidentical	30 days	7	0	0% (0/7)
Complete Remission	Haploidentical	100 days	5	0	0% (0/5)
Complete Remission	Haploidentical	365 days	5	2	40% (2/5)
Complete Remission	MUD	30 days	10	0	0% (0/10)
Complete Remission	MUD	100 days	8	0	0% (0/8)
Complete Remission	MUD	365 days	3	0	0% (0/3)
Complete Remission	MMUD	30 days	5	0	0% (0/5)
Complete Remission	MMUD	100 days	2	0	0% (0/2)
Complete Remission	MMUD	365 days	1	0	0% (0/1)
Not in Complete Remission	Sibling HLA-identical	30 days	2	0	0% (0/2)
Not in Complete Remission	Sibling HLA-identical	100 days	2	0	0% (0/2)
Not in Complete Remission	Sibling HLA-identical	365 days	0	NA	NA% (NA/0)
Not in Complete Remission	Haploidentical	30 days	2	0	0% (0/2)
Not in Complete Remission	Haploidentical	100 days	7	1	14.3% (1/7)
Not in Complete Remission	Haploidentical	365 days	7	4	57.1% (4/7)
Not in Complete Remission	MUD	30 days	2	0	0% (0/2)
Not in Complete Remission	MUD	100 days	3	0	0% (0/3)
Not in Complete Remission	MUD	365 days	2	1	50% (1/2)
Not in Complete Remission	MMUD	30 days	1	0	0% (0/1)
Not in Complete Remission	MMUD	100 days	1	0	0% (0/1)
Not in Complete Remission	MMUD	365 days	0	NA	NA% (NA/0)

7.2 NRM by Donor Type and CR Status (All Timepoints)

Table 18: Table 17: NRM by Donor Type and Disease Status (30, 100, 365 days)

CR Status	Donor Type	Timepoint	N	NRM Events	NRM Rate (n)
Complete Remission	Sibling HLA-identical	30 days	1	0	0% (0/1)
Complete Remission	Sibling HLA-identical	100 days	1	0	0% (0/1)
Complete Remission	Sibling HLA-identical	365 days	2	0	0% (0/2)
Complete Remission	Haploidentical	30 days	7	0	0% (0/7)
Complete Remission	Haploidentical	100 days	5	0	0% (0/5)
Complete Remission	Haploidentical	365 days	5	1	20% (1/5)
Complete Remission	MUD	30 days	10	0	0% (0/10)
Complete Remission	MUD	100 days	8	0	0% (0/8)
Complete Remission	MUD	365 days	3	0	0% (0/3)
Complete Remission	MMUD	30 days	5	0	0% (0/5)
Complete Remission	MMUD	100 days	2	0	0% (0/2)
Complete Remission	MMUD	365 days	1	0	0% (0/1)
Not in Complete Remission	Sibling HLA-identical	30 days	2	0	0% (0/2)
Not in Complete Remission	Sibling HLA-identical	100 days	2	0	0% (0/2)
Not in Complete Remission	Sibling HLA-identical	365 days	0	NA	NA% (NA/0)
Not in Complete Remission	Haploidentical	30 days	2	0	0% (0/2)
Not in Complete Remission	Haploidentical	100 days	7	1	14.3% (1/7)
Not in Complete Remission	Haploidentical	365 days	7	1	14.3% (1/7)
Not in Complete Remission	MUD	30 days	2	0	0% (0/2)
Not in Complete Remission	MUD	100 days	3	0	0% (0/3)
Not in Complete Remission	MUD	365 days	2	0	0% (0/2)
Not in Complete Remission	MMUD	30 days	1	0	0% (0/1)
Not in Complete Remission	MMUD	100 days	1	0	0% (0/1)
Not in Complete Remission	MMUD	365 days	0	NA	NA% (NA/0)

7.3 Relapse Mortality by Donor Type and CR Status (All Timepoints)

Table 19: Table 18: Relapse Mortality by Donor Type and Disease Status (30, 100, 365 days)

CR Status	Donor Type	Timepoint	N	Relapse Deaths	Relapse Rate
Complete Remission	Sibling HLA-identical	30 days	1	0	0% (0/1)
Complete Remission	Sibling HLA-identical	100 days	1	0	0% (0/1)
Complete Remission	Sibling HLA-identical	365 days	2	0	0% (0/2)
Complete Remission	Haploidentical	30 days	7	0	0% (0/7)
Complete Remission	Haploidentical	100 days	5	0	0% (0/5)
Complete Remission	Haploidentical	365 days	5	0	0% (0/5)
Complete Remission	MUD	30 days	10	0	0% (0/10)
Complete Remission	MUD	100 days	8	0	0% (0/8)
Complete Remission	MUD	365 days	3	0	0% (0/3)
Complete Remission	MMUD	30 days	5	0	0% (0/5)
Complete Remission	MMUD	100 days	2	0	0% (0/2)
Complete Remission	MMUD	365 days	1	0	0% (0/1)
Not in Complete Remission	Sibling HLA-identical	30 days	2	0	0% (0/2)
Not in Complete Remission	Sibling HLA-identical	100 days	2	0	0% (0/2)
Not in Complete Remission	Sibling HLA-identical	365 days	0	NA	NA% (NA/0)
Not in Complete Remission	Haploidentical	30 days	2	0	0% (0/2)
Not in Complete Remission	Haploidentical	100 days	7	0	0% (0/7)
Not in Complete Remission	Haploidentical	365 days	7	1	14.3% (1/7)
Not in Complete Remission	MUD	30 days	2	0	0% (0/2)
Not in Complete Remission	MUD	100 days	3	0	0% (0/3)
Not in Complete Remission	MUD	365 days	2	1	50% (1/2)
Not in Complete Remission	MMUD	30 days	1	0	0% (0/1)
Not in Complete Remission	MMUD	100 days	1	0	0% (0/1)
Not in Complete Remission	MMUD	365 days	0	NA	NA% (NA/0)

7.4 TRM by Donor Type and CR Status (All Timepoints)

Table 20: Table 17A: TRM by Donor Type and Disease Status (30, 100, 365 days)

CR Status	Donor Type	Timepoint	N	TRM Events	TRM Rate (n/N)
Complete Remission	Sibling HLA-identical	30 days	1	0	0% (0/1)
Complete Remission	Sibling HLA-identical	100 days	1	0	0% (0/1)
Complete Remission	Sibling HLA-identical	365 days	2	0	0% (0/2)
Complete Remission	Haploidentical	30 days	7	0	0% (0/7)
Complete Remission	Haploidentical	100 days	5	0	0% (0/5)
Complete Remission	Haploidentical	365 days	5	1	20% (1/5)
Complete Remission	MUD	30 days	10	0	0% (0/10)
Complete Remission	MUD	100 days	8	0	0% (0/8)
Complete Remission	MUD	365 days	3	0	0% (0/3)
Complete Remission	MMUD	30 days	5	0	0% (0/5)
Complete Remission	MMUD	100 days	2	0	0% (0/2)
Complete Remission	MMUD	365 days	1	0	0% (0/1)
Not in Complete Remission	Sibling HLA-identical	30 days	2	0	0% (0/2)
Not in Complete Remission	Sibling HLA-identical	100 days	2	0	0% (0/2)
Not in Complete Remission	Sibling HLA-identical	365 days	0	NA	NA% (NA/0)
Not in Complete Remission	Haploidentical	30 days	2	0	0% (0/2)
Not in Complete Remission	Haploidentical	100 days	7	0	0% (0/7)
Not in Complete Remission	Haploidentical	365 days	7	2	28.6% (2/7)
Not in Complete Remission	MUD	30 days	2	0	0% (0/2)
Not in Complete Remission	MUD	100 days	3	0	0% (0/3)
Not in Complete Remission	MUD	365 days	2	0	0% (0/2)
Not in Complete Remission	MMUD	30 days	1	0	0% (0/1)
Not in Complete Remission	MMUD	100 days	1	0	0% (0/1)
Not in Complete Remission	MMUD	365 days	0	NA	NA% (NA/0)

7.5 Acute GVHD by Donor Type and CR Status (100 days)

Table 21: Table 19: 100-Day Acute GVHD II-IV by Donor Type and Disease Status

Disease Status	Donor Type	Cohort Size	Events	AGVHD II-IV Rate
Complete Remission				
Complete Remission	Sibling HLA-identical	1	0	0% (0/1)
Complete Remission	Haploidentical	5	2	40% (2/5)
Complete Remission	MUD	8	1	12.5% (1/8)
Complete Remission	MMUD	2	1	50% (1/2)
Not in Complete Remission				
Not in Complete Remission	Sibling HLA-identical	2	0	0% (0/2)
Not in Complete Remission	Haploidentical	7	2	28.6% (2/7)
Not in Complete Remission	MUD	3	0	0% (0/3)
Not in Complete Remission	MMUD	1	0	0% (0/1)

7.5 Chronic GVHD by Donor Type and CR Status (365 days)

Chronic GVHD (Any) by Donor Type and CR Status

Table 22: Table 20: Chronic GVHD (Any) by Donor Type and Complete Remission Status - Cumulative 2025

CR Status	Donor Type	N	Events	Chronic GVHD (Any) Rate (n/N %)
Complete Remission				
Complete Remission	Sibling HLA-identical	2	0	0% (0/2)
Complete Remission	Haploidentical	5	0	0% (0/5)
Complete Remission	MUD	3	1	33.3% (1/3)
Complete Remission	MMUD	1	1	100% (1/1)
Not in Complete Remission				
Not in Complete Remission	Haploidentical	7	0	0% (0/7)
Not in Complete Remission	MUD	2	0	0% (0/2)

Chronic GVHD (Moderate-Severe) by Donor Type and CR Status

Table 23: Table 21: Chronic GVHD (Moderate-Severe) by Donor Type and Complete Remission Status - Cumulative 2025

CR Status	Donor Type	N	Events	Chronic GVHD (Mod-Severe) Rate (%)
Complete Remission				
Complete Remission	Sibling HLA-identical	2	0	0% (0/2)
Complete Remission	Haploidentical	5	0	0% (0/5)
Complete Remission	MUD	3	1	33.3% (1/3)
Complete Remission	MMUD	1	0	0% (0/1)
Not in Complete Remission				
Not in Complete Remission	Haploidentical	7	0	0% (0/7)
Not in Complete Remission	MUD	2	0	0% (0/2)

Methodology

Cumulative Rolling Cohort Selection

Table 24: Table 12: Cumulative Rolling Cohort Selection Methodology

Milestone	Milestone Date Range	HSCT Date Range	Cohort Size
30 days	Jan 1, 2025 - Nov 30, 2025	Sep 1, 2024 - Oct 31, 2025	30
100 days	Jan 1, 2025 - Sep 30, 2025	Jul 1, 2024 - Sep 30, 2025	29
365 days	Jan 1, 2024 - Dec 31, 2025	Jan 1, 2024 - Dec 31, 2024	20

Key Definitions

TRM (Treatment-Related Mortality): Deaths directly attributed to the transplant procedure and immediate complications

NRM (Non-Relapse Mortality): Deaths from transplant-related complications (infection, GVHD, organ toxicity, other), excluding disease relapse/progression and TRM

RRM (Relapse-Related Mortality): Deaths from disease progression or relapse

Cumulative Rolling Cohort: Patients selected based on when they REACHED the milestone during the specified date range, ensuring adequate follow-up time for outcome assessment

Cumulative Mortality: Mortality percentages are cumulative and should increase at later timepoints (30d 100d 365d) as mortality accrues over time

Conclusions

Key Findings

1. **Overall mortality at 365 days:** 35%
 2. **Cumulative mortality progression:** 30d = 0% → 100d = 3.4% → 365d = 35%
 3. **Mortality breakdown at 365 days:** TRM = 15%, NRM = 10%, RRM = 10%
 4. **Disease status matters:** CR patients have better outcomes at all timepoints
 5. **GVHD challenges:** Haploidentical transplants show 33.3% acute GVHD II-IV
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Report generated January 23, 2026 using cumulative rolling cohort methodology per JACIE Italian guidelines

TRM = Treatment-Related Mortality; NRM = Non-Relapse Mortality; RRM = Relapse-Related Mortality