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# Bridging the Gap: Successful Allogeneic HSCT for Pediatric Inborn Errors of Immunity in a Resource-Constrained Setting

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# Abstract

Allogeneic hematopoietic stem-cell transplantation (HSCT) is curative for many inborn errors of immunity (IEI), but outcomes from single centers in middle-income settings are underreported. We retrospectively studied 34 children who underwent allogeneic HSCT for IEI at a single Mexican referral center between 2016 and 2025; most received a uniform myeloablative regimen with haploidentical donors and post-transplant cyclophosphamide. Overall survival (OS) was estimated by Kaplan–Meier analysis. At a median follow-up of 53 months, 8 patients had died; seven within six months, predominantly from primary graft failure, and one at three years from leukemia. OS was 79.4% at two years and 75.4% at five years. The principal determinant of survival was the achievement of immune reconstitution rather than the underlying diagnosis: OS was 87.7% among reconstituted patients versus 28.6% among those who did not reconstitute ($p < 0.001$), whereas SCID and non-SCID patients did not differ (68.4% versus 81.0%; $p = 0.45$). In this middle-income, single-center cohort, securing and monitoring durable engraftment (not the specific diagnosis) offered the greatest opportunity to improve survival, and HSCT for IEI proved feasible in a resource-constrained setting.


# INTRODUCTION

Inborn errors of immunity (IEI) comprise a diverse group of over 550 distinct disorders caused by pathogenic germline variants. These conditions typically manifest as a spectrum of recurrent or severe infections, autoimmunity, hyperinflammation, and an increased susceptibility to malignancy (1). For many of these patients, particularly those with severe combined immunodeficiency (SCID) and other profound T-cell defects, allogeneic hematopoietic stem cell transplantation (HSCT) remains the only definitive curative therapeutic intervention (2,3).

While outcomes for HSCT in high-income countries have reached overall survival (OS) rates exceeding 90% due to early detection via newborn screening, the landscape in Latin America remains challenging (4,5,6). In middle-income countries like Mexico, clinicians face specific hurdles, including delayed diagnosis, limited access to high-resolution HLA typing, and a high pre-transplant infection burden (7,8). Current literature increasingly emphasizes the need for regional benchmarks to optimize protocols in these settings.

Recent data from emerging economies such as India, Brazil, and Turkey (9,10,11) have demonstrated that survival rates comparable to international standards are achievable through protocol optimization, such as the use of post-transplant cyclophosphamide (PTCy) (12). Reporting the "real-world" experience from large referral centers in Mexico is essential to refine treatment strategies and improve the global mapping of IEI outcomes. We previously reported our institution's initial 11-year experience with 19 children (2008–2018) (7). Here we describe a larger, more recent cohort (2016–2025) transplanted predominantly with haploidentical donors and post-transplant cyclophosphamide, extending and updating that early series.

The primary objective of this study was to determine the success rate and clinical outcomes of pediatric patients with IEI who underwent HSCT at the Unidad Médica de Alta Especialidad No. 25 (IMSS) in Monterrey, Mexico, over a 10-year period (2016–2025). Specific goals included evaluating the OS, distribution of diseases, identification of primary complications, and describing the kinetics of hematopoietic engraftment, chimerism, and immunological reconstitution.

# METHODS

### Study Design and Patient Selection

We conducted a retrospective, observational, and longitudinal study evaluating a cohort of pediatric patients diagnosed with Inborn Errors of Immunity (IEI) who underwent allogeneic hematopoietic stem cell transplantation (HSCT). The study was performed at the Unidad Médica de Alta Especialidad (UMAE) No. 25, Instituto Mexicano del Seguro Social (IMSS) in Monterrey, Mexico, covering 10 years from January 2016 to December 2025. Patients transplanted in 2016–2018 were included in our previous report[7].

Patients were included if they were under 18 years of age and fulfilled the diagnostic criteria established by the European Society for Immunodeficiencies (ESID). Phenotypic classification of the underlying immunodeficiencies was updated in accordance with the 2024 International Union of Immunological Societies (IUIS) criteria.

### Exclusion Criteria

To ensure data robustness, we excluded patients with a follow-up period of less than one year, those with irregular or incomplete medical records, and infants diagnosed with transient hypogammaglobulinemia of infancy. Patients diagnosed with bone marrow failure syndromes, autoinflammatory diseases, or phenocopies of congenital immune defects were also excluded, as these individuals were primarily referred to specialized hematology, rheumatology, or adult clinics rather than the pediatric immunology department.

### Conditioning and GvHD Prophylaxis Protocols

Most patients (n=30) received a uniform myeloablative conditioning regimen consisting of busulfan (16 mg/kg total, administered in 16 doses every 6 hours over days −6 to −3), cyclophosphamide (60 mg/kg/day, days −3 to −2), and rabbit anti-thymocyte globulin (thymoglobulin, 2.5–3 mg/kg/day, days −6 to −4). Intravenous immunoglobulin was given on day −1 at the patient's established replacement dose (600–800 mg/kg) and continued weekly until neutrophil engraftment, after which its frequency was tapered to monthly through the first post-transplant year. Day 0 was defined as the day of stem-cell infusion; conditioning days are numbered negatively. A minority of patients received myeloablative variants of this backbone according to drug availability; two children with hemophagocytic-spectrum disease (Griscelli and Chédiak–Higashi syndromes) additionally received etoposide (150 mg/m²/day,

days −6 to −3), and one patient with donor-specific anti-HLA antibodies underwent pre-transplant desensitization with plasmapheresis and rituximab.

GVHD prophylaxis was based on post-transplant cyclophosphamide (50 mg/kg/day on days +3 and +4), a calcineurin inhibitor (tacrolimus 0.25 mg/kg/d, serum target level 6-8ng/ml; or cyclosporine 2.5mg/kg/d, target 200-300ng/ml) from day +5 to +365, tapered from month +6 in the absence of chronic GVHD, and mycophenolate mofetil (1200 mg/m²/day) from day +5 to day +35. Engraftment was defined as the number of days from stem cell infusion (day 0) to the first of three consecutive days when blood cell counts (neutrophils >500/ml, platelets >20,000/ml) reach and maintain the target level without a transfusion. Chimerism was defined as the proportion of donor-derived hematopoietic stem cells detected in the recipient's blood on days 45, 100, and 365. Immune reconstitution was defined as an absolute cell count of >500 neutrophils/ml, >300 CD3+ T lymphocytes/ml, >200 T CD4+ lymphocytes/ml, over 100 CD19+ B cells/ml, and serum IgG levels >500 mg/dl without administration in the preceding 4 weeks.

**Data Collection and Definitions**

Medical records were comprehensively reviewed to extract demographic data (sex, age at disease onset, age at presentation, and history of consanguinity), anthropometric measurements at diagnosis, and specific genetic and laboratory findings.

Transplant-specific variables were recorded, including engraftment and reconstitution: neutrophil and platelet engraftment kinetics, alongside the achievement of full immunological reconstitution; chimerism: donor chimerism percentages evaluated at predefined post-transplant intervals; complications: incidence and severity of acute and chronic Graft-versus-Host Disease (GvHD), as well as clinical evolution and primary causes of mortality.

**Statistical Analysis**

Data distribution normality was assessed using the Shapiro–Wilk test using SPSS v22.0 (IBM Corp, Armonk, NY). Continuous variables with normal distributions are expressed as means and standard deviations (SD), whereas non-normally distributed data are reported as medians with interquartile ranges (IQR). Categorical variables are presented as absolute numbers and percentages (n, %).

Overall survival (OS) was defined as the time from stem-cell infusion (Day 0) to death from any cause; surviving patients were censored at the date of last contact (data cutoff, 24 June 2026). OS was estimated by the Kaplan–Meier method with 95% confidence intervals (Greenwood's formula), and median follow-up was estimated by the reverse Kaplan–Meier method. Survival between subgroups was compared using the log-rank test, and the association between immune reconstitution and mortality was assessed with Fisher's exact test. A two-sided $p < 0.05$ was considered significant. Survival analyses were performed in Python 3 (lifelines 0.30, Python Software Foundation, python.org).

**Ethical Considerations**

The study protocol was approved by the Local Ethics and Research Committee of UMAE 25. Written informed consent was obtained from all patients' parents or legal guardians before data extraction, and all procedures were conducted in accordance with the principles of the Declaration of Helsinki.

# RESULTS

### Patient Characteristics and IEI Subtypes

Thirty-four children underwent allogeneic HSCT for IEI between 2016 and 2025. The median age at transplantation was 34.7 months (range, 2–156), and 21 (61.8%) were male. The most frequent diagnosis was SCID (12; 35.3%), followed by Wiskott–Aldrich syndrome (8; 23.5%) and chronic granulomatous disease (5; 14.7%); **Table 1** shows the full distribution. Most transplants were haploidentical (29; 85.3%), and 23 patients (67.6%) were CMV-IgG seropositive before transplantation. At last follow-up, 26 patients (76.5%) were alive, and 8 (23.5%) had died.

### Hematopoietic Engraftment and Immunological Reconstitution

Sustained hematopoietic engraftment was attained in 91% (31/34). Successful immunological reconstitution was achieved in 27 out of the 34 treated patients (79.4%), with a mean infused CD34+ cell dose of 52.5 x$10^6$/kg (range 4.1-145). Conversely, lack of immune reconstitution was documented in 7 cases (20.6%), predominantly clustered within the SCID subcohort (n=4) and Griscelli syndrome (n=2), alongside a single case of CGD. Primary graft failure occurred in 3 patients.

Full chimerism (>99%) was reported in 76.7% at 3 months, 77.8% at 6 months, and 84.6% at 12 months.

### Overall Survival Analysis

The survival rate for the entire cohort was 76.5%, with an overall mortality rate of 23.5% (n=8) over the 10-year study period. A critical pathophysiological distinction was observed among the causes of mortality. Five of the eight recorded deaths (62.5%) occurred in patients who suffered from early primary graft failure and its immediate infectious or metabolic sequelae. In contrast, 3 patients (diagnosed with SCID, DOCK8 deficiency, and WAS) successfully achieved initial immunological reconstitution but succumbed to late-onset transplant-related complications, such as severe opportunistic infections or Graft-versus-Host Disease (GvHD).

At a median follow-up of 53 months (survivors: median 52, IQR 33–92), 8 of 34 patients had died. Seven deaths occurred within the first six months after infusion — five within the first month (including one on day 17) and one each at months 4 and 6 — while a single late death occurred at three years, secondary to acute lymphoblastic leukemia in a patient who had achieved immune reconstitution. By Kaplan–Meier analysis, overall survival fell to 79.4% by month 6 and remained stable until the late event at month 36, after which it settled at 75.4% (95% CI, 56.6–87.0) through the end of follow-up; median OS was not

reached (**Figure 1**). Estimated OS was 79.4% at 1 and 2 years and 75.4% at 5 years, with 27, 22, and 10 patients at risk, respectively; only one patient was followed to 10 years.

### Immune reconstitution and mortality

Failure to achieve immune reconstitution was the principal determinant of mortality: five of 7 patients who did not achieve reconstitution died (71.4%), compared with 3 of 27 with reconstitution (11.1%; Fisher's exact p = 0.004; odds ratio ≈ 20). By Kaplan–Meier analysis, estimated OS was 28.6% without reconstitution versus 87.7% with reconstitution (log-rank $\chi^2 = 17.1$, $p < 0.001$; **Figure 2**). The five deaths in the non-reconstituted group all occurred within the first month and were attributable to primary graft failure and its immediate infectious or metabolic sequelae, whereas two of the three reconstituted deaths occurred at months 4 and 6 (opportunistic infection or GVHD) and one at three years (ALL). See also **Table 2**.

### Survival by diagnosis

Overall survival did not differ significantly between patients with SCID (including RAG1-deficient Omenn syndrome; n=13, OS 68.4%) and those with other IEI (81.0%, 17/21; log-rank p = 0.45; **Figure 3**), indicating that immune reconstitution, rather than the underlying diagnosis, governed survival in this cohort.

## DISCUSSION

In this single-center cohort of 34 children with inborn errors of immunity (IEI) undergoing allogeneic hematopoietic stem-cell transplantation (HSCT) over a 10-year period, overall survival was 76.5%, and the principal determinant of survival was not the underlying diagnosis but whether the child achieved immune reconstitution. Survival reached 87.7% among patients who reconstituted, versus 28.6% among those who did not (log-rank $p < 0.001$); a gap mirrored in mortality (11.1% vs 71.4%; Fisher's exact p = 0.004; odds ratio ≈ 20). By contrast, survival did not differ significantly between patients with SCID and those with other IEI (68.4% vs 81.0%; p = 0.45). Mortality was early and tightly clustered: seven of the eight deaths occurred within the first six months, five within the first month, and were driven overwhelmingly by primary graft failure and its immediate sequelae in patients who never reconstituted. One reconstituted patient died at three years from ALL; aside from that single late event, survival was durable after the early period. The central message of this cohort is therefore that engraftment and immune reconstitution (an early, measurable outcome) separated survivors from non-survivors far more sharply than the diagnostic label.

The strengths of this study include a real-world cohort spanning a full decade at a single middle-income center — a setting under-represented in the transplant literature — with complete outcome ascertainment in which every death was individually dated, so that no survival time required imputation.

Several limitations temper the conclusions. The sample is small (n = 34), which limits statistical power; in particular, the non-significant comparison between SCID and non-SCID patients should be read as

inconclusive rather than as evidence of no difference. The study is single-center and retrospective; although most patients received a uniform myeloablative, busulfan-based regimen, a minority received variants according to drug availability, introducing some heterogeneity in conditioning. Median follow-up was moderate (53 months), and only one patient reached 10 years, so very long-term inferences are limited. Finally, immune reconstitution is assessed after transplantation and lies partly on the causal pathway to survival: a patient must survive the early post-transplant period to be classified as reconstituted, so the reconstitution/survival association is susceptible to guarantee-time (immortal-time) bias and should be interpreted as associative rather than causal. The mechanistic coherence of the data (the deaths in the non-reconstituted group were driven by graft failure) argues that this association reflects underlying biology rather than statistical artifact alone.

Our overall survival of 76.5% compares favorably with contemporary series from middle-income and developing country programs. Multicenter cohorts from Brazil (n = 221; 5-year OS 71.6%) [10] and India (n = 228; 5-year OS 68%, improving from 26.8% to 77.5% in the post-2016 era) [9], together with a single-center Jordanian series reporting a 2-year OS of 93% [13], place our results within, and toward the upper part of, the range achievable outside high-resource registries; dedicated national experiences from Turkey [11], Japan (n = 567) and Russia (n = 454), the latter two reporting overall survival near 74% [15, 16], further define this range. In high-income registries, survival now generally exceeds 80% [17] and reaches approximately 90% or higher for matched-sibling-donor SCID [4], while a 32-year single-center European experience reported a 5-year OS of 83.4% [18].

Notably, several of the middle-income series that most resemble ours: haploidentical transplantation with post-transplant cyclophosphamide from Brazil (2-year OS 66%) [19], India [20], and Jordan [13], share our donor and GVHD prophylaxis platform, strengthening the comparison. Two features of our cohort echo this literature. First, mortality was early and dominated by infection and graft failure, mirroring haploidentical/post-transplant-cyclophosphamide series in which most deaths occurred within the first months [9, 19]. Second, the primacy of immune reconstitution as a survival determinant is consistent with prospective consortium data, in which early post-transplant T cell recovery (6-month CD4 + counts and day-100 T cell markers) predicted survival and long-term reconstitution [14, 21]. Our finding that reconstitution, rather than the underlying diagnosis, stratified survival extends this principle to a heterogeneous, single-center IEI program in a middle-income setting.

Because reconstitution declares itself early and can be monitored directly, it functions as an actionable clinical checkpoint: the child who fails to engraft or reconstitute is identifiable within weeks to months and belongs to a group whose survival without intervention is poor. This argues for intensive early surveillance (serial chimerism and immune monitoring), prompt recognition of impending graft failure, and readiness to act (second procedures, stem-cell boosts, or donor lymphocyte infusions where appropriate). Because most deaths were early and graft-failure driven, the largest survival gains are likely to come from safeguarding primary engraftment: through donor selection, optimization of conditioning intensity and serotherapy, and management of donor-specific antibodies, in line with EBMT/ESID

recommendations (3); rather than from diagnosis-specific tailoring alone. Finally, the cohort demonstrates that a sustained, effective HSCT program for IEI is feasible in a middle-income setting.

Prospective, multicenter collaboration, for which regional registries such as LASID are well suited, would provide the numbers needed to test the diagnosis- and conditioning-specific effects that the present cohort is underpowered to resolve. Moving from a binary reconstitution endpoint to time-resolved measures (T-cell and TREC kinetics, thymic output, and chimerism trajectories), and modeling reconstitution as a time-dependent covariate, would sharpen prediction and directly address the guarantee-time bias noted above [18, 19]. Systematic evaluation of the myeloablative busulfan–cyclophosphamide/ATG regimen with post-transplant cyclophosphamide, specifically its effect on engraftment and immune recovery, is a natural next step.

To conclude, in children undergoing allogeneic HSCT for IEI, achieving immune reconstitution —not the specific underlying diagnosis— was the principal determinant of survival: excellent in those who reconstituted and poor in those who did not, with death driven overwhelmingly by early graft failure. Clinical efforts and resources are therefore best directed at securing, monitoring, and protecting durable engraftment and immune recovery. These single-center results from a middle-income setting affirm both the value of HSCT for IEI and the imperative to safeguard the early graft.

# Declarations

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**Conflicts:** The authors declare no competing interests.

**Data availability:** The data that support the findings of this study are available from the corresponding author upon reasonable request.

**Ethics approval and consent to participate:** This study was approved by the Local Committees for Research and Ethics in Health of the Instituto Mexicano del Seguro Social, Hospital de Especialidades UMAE No. 25 (institutional registration R-2025-1901-116). Owing to the retrospective design and the use of de-identified data abstracted from clinical records, the committees waived the requirement for individual informed consent. The study was conducted in accordance with the Declaration of Helsinki.

**Author contributions:** ATSB conceived and coordinated the study, supervised the project, acquired funding, and contributed to clinical care and to the interpretation of results. ASG wrote the thesis on

which this work is based and contributed to data collection. YSG contributed to data collection and to data verification. EVM, DMZ, JAGC, LMSS and AJRV contributed to clinical care and data collection. HRD and MGGV were responsible for transplantation care and conditioning and contributed to data analysis and patient follow-up. DCCF supervised the thesis, was responsible for transplantation care and conditioning, contributed to data analysis, interpretation of results and patient follow-up, and critically revised the manuscript for important intellectual content. SOLR conceived the study, drafted the manuscript, and performed the statistical analysis and interpretation of results. All authors read, revised critically, and approved the final manuscript.

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# Tables

**Table 1.** Baseline characteristics of the cohort (n = 34).

| Characteristic | n = 34 |
|---|---|
| Age at transplant, months — median (range) | 34.7 (2–156) |
| **Sex, n (%)** | |
| Male | 21 (61.8) |
| Female | 13 (38.2) |
| **Diagnosis, n (%)** | |
| SCID | 12 (35.3) |
| Wiskott–Aldrich syndrome | 8 (23.5) |
| Chronic granulomatous disease | 5 (14.7) |
| Griscelli syndrome type II | 2 (5.9) |
| DOCK8 deficiency (hyper-IgE) | 2 (5.9) |
| Omenn syndrome | 1 (2.9) |
| Chédiak–Higashi syndrome | 1 (2.9) |
| Dyskeratosis congenita | 1 (2.9) |
| Severe congenital neutropenia (Kostmann) | 1 (2.9) |
| Immuno-osseous dysplasia | 1 (2.9) |
| **Donor type, n (%)** | |
| Haploidentical | 29 (85.3) |
| HLA-matched | 5 (14.7) |
| CMV IgG seropositive, n (%) | 23 (67.6) |
| **Vital status at last follow-up, n (%)** | |
| Alive | 26 (76.5) |
| Deceased | 8 (23.5) |

*SCID, severe combined immunodeficiency; CMV, cytomegalovirus. Omenn syndrome was caused by RAG1 deficiency and was grouped with SCID for the survival analysis.*

**Table 2.** Overall mortality and its association with immune reconstitution.

| Outcome | Reconstitution achieved (n=27) | No reconstitution (n=7) | Total (n=34) |
|---|---|---|---|
| Alive | 24 (88.9%) | 2 (28.6%) | 26 (76.5%) |
| Deceased | 3 (11.1%) | 5 (71.4%) | 8 (23.5%) |

Fisher's exact p = 0.004; odds ratio ≈ 20. Timing of death: seven of the eight deaths occurred within the first six months; five within the first month (including one on day 17), one each at months 4 and 6, and one at three years (acute lymphoblastic leukemia).

## Figures

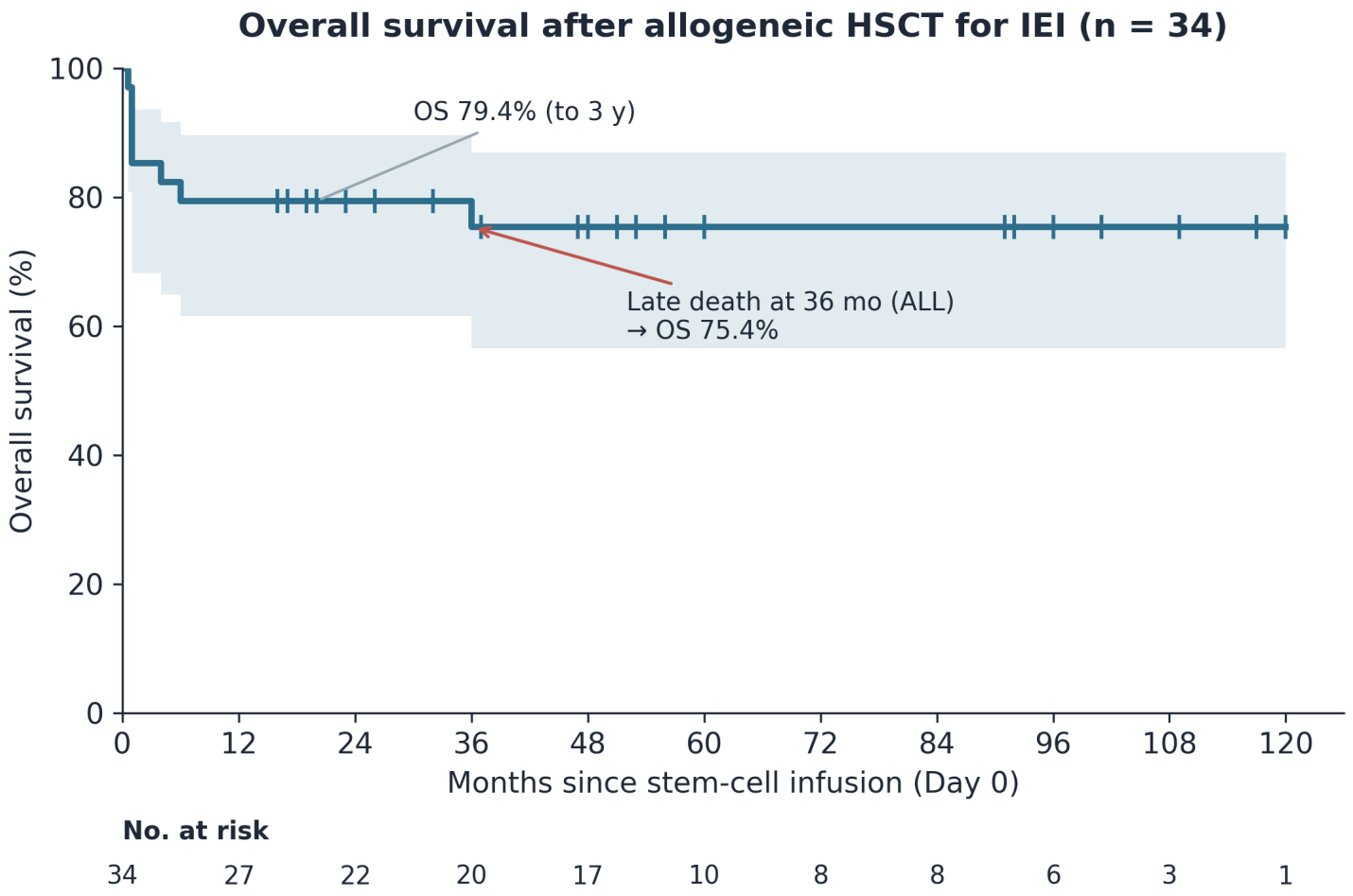



**Figure 1**

Kaplan–Meier overall survival for the entire cohort (n = 34) after allogeneic HSCT. Tick marks denote censored patients; the shaded band is the 95% confidence interval; the number at risk is shown below the axis. Overall survival was 79.4% through the first three years; a single late death at 36 months (ALL) lowered it to 75.4%. Median OS was not reached.

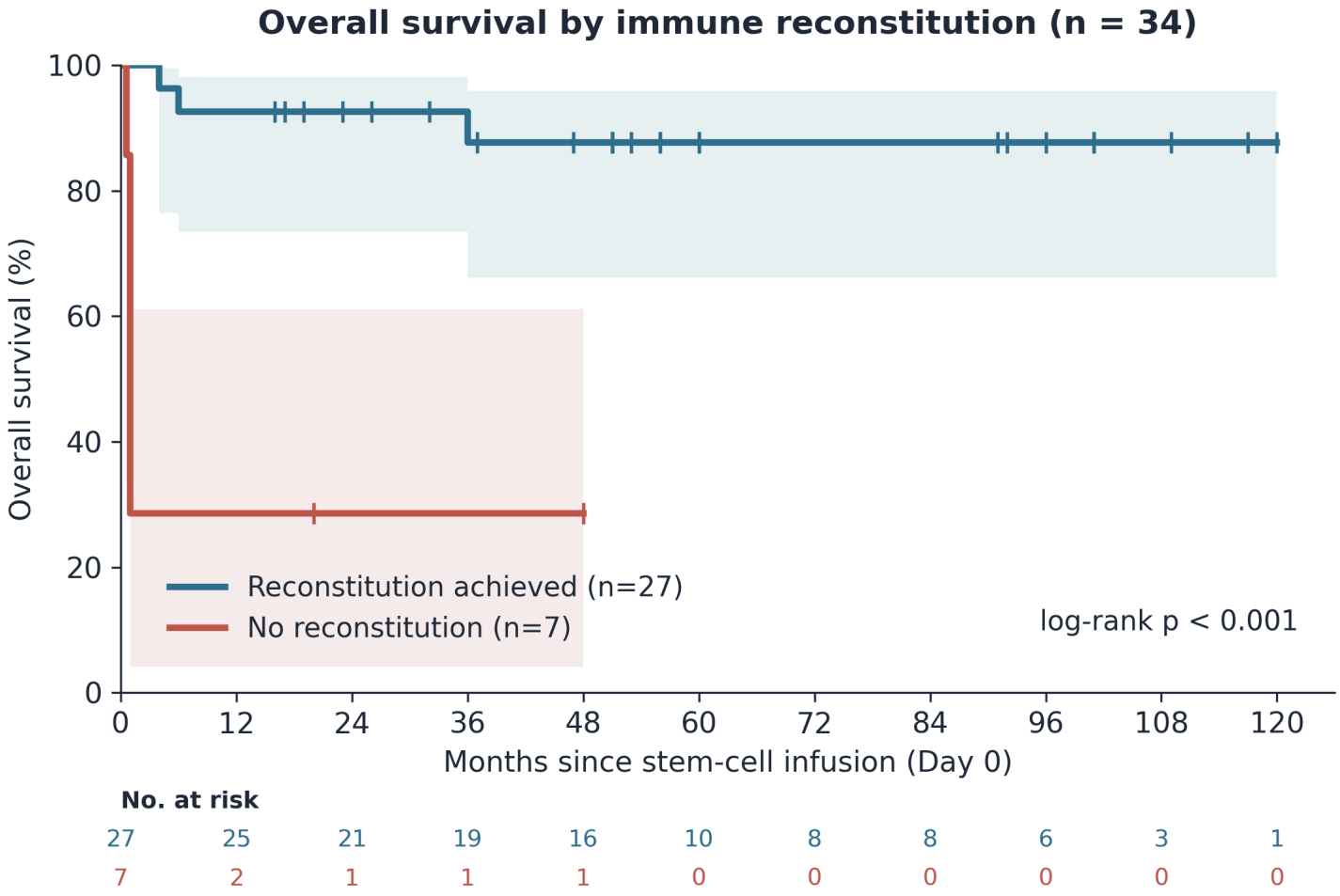



**Figure 2**

Kaplan–Meier overall survival stratified by achievement of immune reconstitution. Patients achieving reconstitution (n = 27) had markedly superior survival (87.7%) compared with those who did not (n = 7; 28.6%); log-rank p < 0.001.

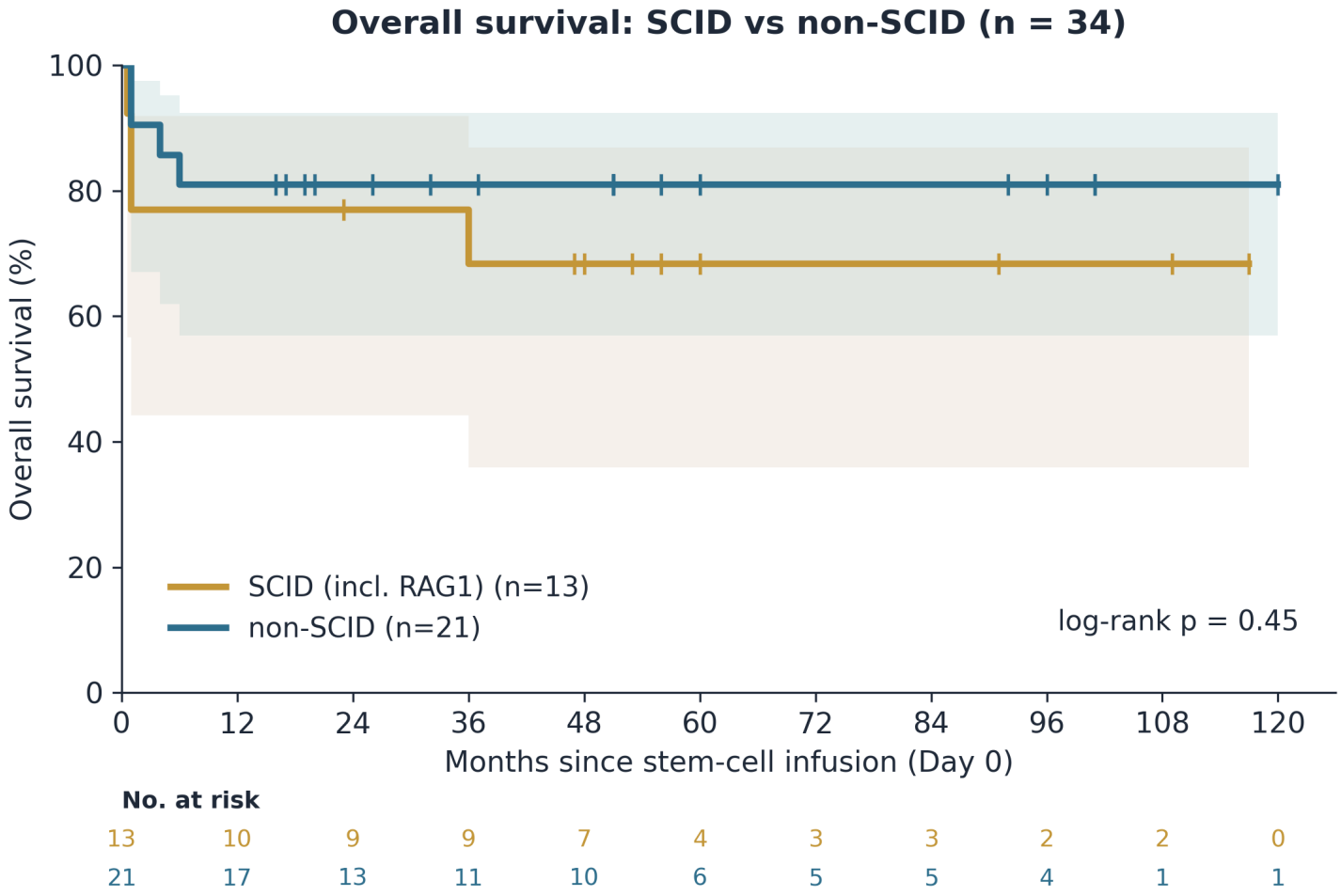



**Figure 3**

Kaplan–Meier overall survival for SCID (n = 13) versus non-SCID IEI (n = 21). Survival did not differ significantly (log-rank p = 0.45).