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REVIEW ARTICLE

Hematopoietic stem cell transplantation in Wiskott–Aldrich syndrome: A systematic review and meta-analysis

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Abstract

Background: Wiskott–Aldrich syndrome (WAS) is a rare X-linked primary immunodeficiency that typically presents early in life with recurrent infections, eczema, and microthrombocytopenia. Patients are also at increased risk of autoimmune complications and malignancies. In recent years, hematopoietic stem cell transplantation (HSCT) has emerged as a potentially curative treatment option. **Aim:** To systematically review and meta-analyze the available evidence on the safety and effectiveness of HSCT in patients with WAS. **Methods:** This systematic review and meta-analysis followed the Preferred Reporting Items for Systematic Reviews and Meta-Analyses guidelines (PROSPERO: CRD42025640272). We searched four databases (Cochrane CENTRAL, ClinicalTrials.gov, PubMed/MEDLINE, and ScienceDirect) for relevant studies from inception through May 2024. We included observational studies and clinical trials, including those involving WAS patients undergoing HSCT. Outcomes of interest included overall mortality and survival, event-free survival, graft-versus-host disease (GVHD), autoimmunity, symptomatic recovery, engraftment, and chimerism. **Results:** We analyzed 24 studies, pooling 884 HSCT-recipient WAS patients. Most studies were of low methodological quality (83%). The pooled overall mortality was 13% (95% confidence interval = 9.5%–16.5%), with infections being the most common cause of death (63.6%), followed by GVHD (24.1%). Event-free survival and symptomatic recovery were 84.6% and 72%, respectively. Mean neutrophil and platelet engraftment occurred at 18.75 and 31.74 days post-HSCT, respectively. Full and mixed chimerism was achieved in 72.5% and 18.9% of cases, respectively. Selected complications included acute GVHD (35.9%), chronic GVHD (11.9%), and autoimmune manifestations (19%). **Conclusion:** HSCT is associated with favorable survival and clinical outcomes in patients with WAS; however, treatment-related mortality remains substantial, with infections and GVHD reported as the leading causes of mortality among HSCT recipients. **Relevance for patients:** This study helps patients and families weigh the favorable survival and

symptom recovery associated with HSCT against potential risks such as infections, graft-versus-host disease, and autoimmune complications.

Keywords: Heart; Hematopoietic stem cells; Stem cells; Wiskott–Aldrich syndrome

1. Introduction

Wiskott–Aldrich syndrome (WAS) is a rare X-linked primary immunodeficiency with an estimated incidence of 1 in every 100,000 live births.[1] It results from a mutation in the structural gene encoding WAS protein (*WASp*) located at Xp11.22–23.[2] The disease has a broad spectrum of phenotypes, depending on the mutation, ranging from milder forms such as X-linked thrombocytopenia and X-linked neutropenia to more severe phenotypes, such as classic WAS.[1,2] Milder cases of WAS are typically managed conservatively with supportive care and intensive monitoring.[3] Classic WAS in early childhood is classically presented with a triad of recurrent infections, eczema, and microthrombocytopenia that can progress to involve complications such as autoimmunity and malignancies.[3,4] Historically, clinicians treated classic WAS with splenectomy to improve thrombocytopenia and immunoglobulin therapy to prevent recurrent infections.[5]

Although supportive therapies remain the mainstay, hematopoietic stem cell transplantation (HSCT) and autologous gene therapy (GT; for patients without suitable donors) have emerged as standard, widely accepted curative treatments for various inborn errors of immunity in a select group of patients.[6] Because *WASp* is constitutively expressed only in lineages derived from hematopoietic stem cells (HSCs), several clinical studies have shown that HSCT is well suited to correct both hematologic and immunologic defects associated with WAS.[7–10] In the absence of curative therapies, affected patients experience diminished life expectancy and an impaired quality of life due to recurrent infections, bleeding complications, and autoimmune issues, which may culminate in malignancies.[11,12] As the primary curative therapy for WAS, HSCT addresses the underlying immunodeficiency and thrombocytopenia by restoration of hematopoiesis and immune function. HSCT outcomes have substantially improved over time, largely due to donor selection with human leukocyte antigen (HLA)-matched sibling and unrelated donors and conditioning regimens with superior supportive care and cell delivery strategies.[13]

Despite the reported success of HSCT for patients with WAS, several concerns regarding overall safety and effectiveness have emerged. Numerous studies have highlighted the risk of developing complications following HSCT.[13,14] HSCT performed at a younger age has been associated with better outcomes, supporting the recommendation for early transplantation.[13] However, other studies have shown that adult patients can also achieve favorable outcomes with HSCT.[15] When matched donors are unavailable for allogeneic HSCT, alternative stem cell sources have been suggested. Autologous HSCs modified *ex vivo* through GT have been proven to be an effective alternative.[16] Cord blood transplantation is also a viable option.[17] Additionally, studies have emphasized the utility of HSCs from haploidentical family donors, particularly when pre- and post-transplantation prophylactic measures are implemented.[18] Immunosuppression and myeloablative conditioning have been the standard preparative regimen for HSCT in WAS.[17] To achieve desirable outcomes with minimal adverse effects, researchers have proposed various conditioning regimens.[17–19] Differences in conditioning regimens across studies have led to variations in chimerism status, overall survival, and the incidence of events such as graft-versus-host disease (GVHD), autoimmunity, infections, and mortality following transplantation.[20,21]

To the best of our knowledge, this is the first meta-analysis to evaluate HSCT outcomes in patients with WAS. This study aims to synthesize available quantitative evidence on the safety and clinical outcomes of HSCT in patients with WAS, support evidence-based clinical decision-making, and identify areas for future research.

2. Methods

2.1. Protocol and registration

This systematic review and meta-analysis was conducted in accordance with the Preferred Reporting Items for Systematic Reviews and Meta-Analyses guidelines. We developed a protocol and submitted it prospectively to the International Prospective Register of Systematic Reviews (PROSPERO [CRD42025640272]).

2.2. Literature search strategy and eligibility criteria

To identify all available and relevant studies, we systematically searched four databases: Cochrane

CENTRAL, ClinicalTrials.gov, PubMed/MEDLINE, and ScienceDirect. We searched from inception through May 2024. Two authors (MJ, IS) conducted the searches. Two different search strategies were deployed: (i) using the Medical Subject Headings keywords and (ii) using text field searching. The following keywords were used to identify the relevant studies using the Boolean operators: ((Wiskott-Aldrich syndrome [Title/Abstract]) OR (Eczema-thrombocytopenia-immunodeficiency syndrome[Title/Abstract]) OR (Wiskott syndrome [Title/Abstract])) AND ((stem cell[Title/Abstract]) OR (precursor cell[Title/Abstract])). We adjusted these strategies to each database's search style.

This meta-analysis included studies fulfilling the following criteria: (i) observational studies (retrospective or prospective) or clinical trials (randomized or non-randomized); (ii) included participants with an established diagnosis of WAS; (iii) analyzed patients undergoing HSCT (including engineered gene-edited autologous HSCT); and (iv) described relevant outcomes of interest. The study excluded isolated case reports/series, letters to the editor, editorials, reviews, non-English studies, studies with non-relevant outcomes, and interventions other than HSCT. When an included study reported a broader cohort in which only a subset of participants underwent the intervention of interest, we included only data from the treated subset in the quantitative synthesis.

2.3. Data extraction

After fully screening all included studies, we extracted data from eligible studies (EA, YA, and OE) and used a fourth tiebreaker (IS). The extraction was achieved using a predefined sheet that incorporated the following domains: main characteristics of the studies (i.e., first author's name, year of publication, country, and study design), patient baseline characteristics (i.e., sample size, gender, mean age, and WAS score), details of the intervention (i.e., cell type, cell source, donor if applicable, and follow up period), and the outcomes of the intervention (i.e., overall mortality and the reported cause of death, overall survival, disease duration, GVHD, and chronic GVHD rate, post-HSCT autoimmune manifestation, and symptomatic recovery rate).

2.4. Quality assessment

Three reviewers (OE, YA, and MJ) independently assessed the quality of all the included studies. Observational studies were evaluated using the Newcastle-Ottawa Scale (NOS), which assessed the following three domains: (i) selection (sample representativeness, selection of the non-exposed cohort, ascertainment of the exposure, and demonstration that the outcome of interest was not present at the start of the study), (ii) comparability, and (iii) outcome (outcome assessment method, follow-up time, and adequacy). We considered a study of good quality if it had 3 or 4 stars in the selection domain, 1 or 2 stars in the comparability domain, and 2 or 3 stars in the outcome/exposure domain.[22] Meanwhile, clinical trial studies were evaluated using the Risk of Bias Assessment Tool for Nonrandomized Studies version 2 (RoBANS 2), which incorporated an 8-domain methodological assessment checklist that included (i) comparability of the target group, (ii) target group selection, (iii) confounders, (iv) measurement of intervention or exposure, (v) blinding of assessors, (vi) outcome assessment, (vii) incomplete outcome data reporting, and (viii) selective outcome reporting. For each domain, independent assessors assigned a risk-of-bias score of "low," "high," or "unclear," according to the specified criteria.[23]

2.5. Outcome definition

This meta-analysis assessed the following outcomes: (i) overall mortality and survival, (ii) rate of GVHD and chronic GVHD, (iii) autoimmune manifestations, (iv) symptomatic recovery rate, (v) days to platelet and neutrophil engraftment, and (vi) rates of full and mixed chimerism. We coded data from eligible studies in binary format for analysis, except for days to platelet and neutrophil engraftment, which we recorded as continuous outcomes. We conducted a single-arm meta-analysis, and we performed data synthesis and analysis using OpenMeta[Analyst]© software from Brown University. We computed the prevalence of all outcomes (i.e., overall mortality/survival and cause of death, GVHD rate and chronic GVHD, autoimmune manifestations, and symptomatic recovery rate) from absolute numbers reported by each study. We selected a random-effects model because of the high variability among the included studies. We performed subgroup analyses by study design (i.e., observational and non-randomized clinical trials) and study quality (i.e., low and high risk of bias) to identify sources of heterogeneity. We evaluated heterogeneity using the I^2 statistic, with ≤25%, 26%–74%, and ≥75% indicating low, moderate, and high heterogeneity, respectively. Furthermore, we performed leave-one-out analyses to address heterogeneity. A p-value of <0.05 was considered significant at a two-tailed 95% confidence interval (CI). When mean and standard deviation (SD) were not available, we used the methods suggested by Hozo *et al.*[24] to estimate them from the reported median, range, and sample size. We then incorporated the estimated values into the meta-analysis.

3. Results

3.1. Study selection

The database searches retrieved 451 records. Duplicate records were removed (n = 28), with a further 346 records excluded after title/abstract screening, and 53 after full-text review (Figure 1). A total of 24 studies were eligible for this systematic review and meta-analysis.[8,13,19,21,25–44]

3.2. Characteristics of included studies

Eligible studies were published between 2002 and 2022 and were conducted across different countries: Italy (n = 2),[26,37] Japan (n = 4),[25,27,35,38] the United States of America/Canada (n = 2),[8,13] France/United Kingdom (n = 2),[31,44] multi-city Europe (n = 2),[21,43] Türkiye (n = 2),[40,41] Germany (n = 2),[30,34] India (n = 2),[36,42] Australia and New Zealand (n = 1),[32] Brazil (n = 1),[39] Israel (n = 1),[33] Russia (n = 1),[19] Saudi Arabia (n = 1),[29] and Spain (n = 1).[28]Observational cohort studies accounted for 87.5% of the total included studies (n = 21), while the remaining studies were non-randomized clinical trials (n = 3) (Table 1).

This meta-analysis pooled data from 884 male patients, most of whom were younger than three years at HSCT. All underwent HSCT (n = 884). The WAS scores varied across studies, as reported by 13 studies as follows: I (n = 4), II (n = 24), I–II (n = 77), II–III (n = 44), III (n = 47), III–IV (n = 114), IV (n = 60), IV–V (n = 5), and V (n = 114), while some studies did not report on the WAS score. Most studies used bone marrow-derived HSCs, while others used umbilical cord or peripheral blood. Three studies used autologous cells; most used matched related donor cells. Few studies used mismatched cells from unrelated donors. Follow-up duration varied across studies, ranging from as short as three months to more than 100 months.

3.3. Outcome analysis

3.3.1. Mortality and survival

All 24 studies, comprising 884 patients, reported the mortality and survival outcomes. The pooled mean prevalence of overall mortality was 13% (95% CI = 9.5%–16.5%), with reporting studies showing an overall

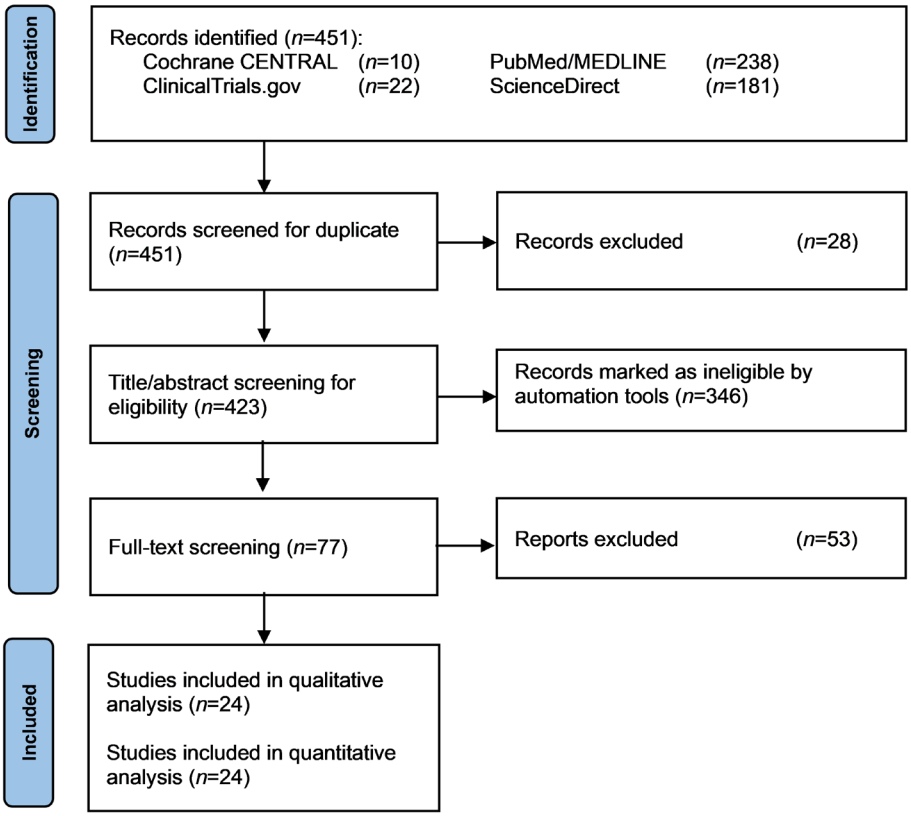


Figure 1. Preferred Reporting Items for Systematic Reviews and Meta-Analyses flowchart

Table 1. Baseline characteristics of included studies

Author (Year)	Country	Study design	Sample size (*n*)	Mean age ± SD	WAS score	Cell type	Cell source	Donor	Follow-up duration
Yamaguchi *et al.* (2002)[35]	Japan	Retrospective	12	20.4 months	NA	HSC	BM - CB	URD, RD	6 to 30 months
Pai *et al.* (2006)[26]	Italy	Retrospective	23	11.1 ± 80.1 months	II (6), III (2), II–III (3), IV (1), V (11)	HSC	BM - CB	RID: 5, MMRD: 2, closely MUD: 16	3–132 months
Tsuji *et al.* (2006)[27]	Japan	Retrospective	11	50 months	NA	HSC	BM - CB	5 RD, 6 UR	168 months
Muñoz *et al.* (2007)[28]	Spain	Retrospective	13	30 months	IV (12), V (1)	HSC	BM	3 MSD, 7 URD, 2 MMUD, 1 haploidentical	101 months
Ozsahin *et al.* (2007)[21]	Multi-city Europe	Retrospective	96	24 months	II–III (41), IV–V (5)	HSC	BM & PBSC, CB	MSD: 45, URD: 32, MMRD: 19	300 months
Al-Ghonaium *et al.* (2008)[29]	Saudi Arabia	Retrospective	14	NA	NA	HSC	BM - CB H	10 RD, 4 UD	NA
Friedrich *et al.* (2008)[30]	Germany	Retrospective	39	20.4 months	NA	HSC	NA	HLA-ID UD: 15, non-ID PD: 15, MSD: 9	132 months
Mahlaoui *et al.* (2013)[31]	France	Retrospective	160 (22 received HSCT)	0.8 years	V (26). Other scores not specified	HSC	UCB (2). Other source not specified	MUD, MSD, MRD, MMUD, haploidentical	6 years (1.5–2.6 years)
Mitchell *et al.* (2013)[32]	Australia and New Zealand	Retrospective	27	NA	NA	HSC	BM & PBSC, CB	NA	60 months
Stepensky *et al.* (2013)[33]	Israel	Retrospective	14	3.25 years (0.16–6.6 years)	IV (7), V (7)	HSC	BM, UCB, PBSC	MUD, MSD, MFD	3.4 years (5–12.5 years)
Braun *et al.* (2014)[34]	Germany	Non-randomized clinical trial	10	5.1 ±4.01 years	III (2), V (8)	HSC GT	PBSC (mononuclear)	Autologous, GT	47.4 ± 20.65 months
Oshima, *et al.* (2015)[25]	Japan	Retrospective	24	7.5 years (range 1.3–37 years)	I & II (NS) V (3)	HSC	BM, UCB	MUD	50 months (21–204 months)
Ngwube *et al.* (2017)[8]	United States	Retrospective	12	13.8 ± 10.87 months	II (6), III (2), IV (3), V (1)	HSC	BM, mobilized PB	4 MSD, 5 MUD, 3 SAMUD	67 months
Balaschov *et al.* (2018)[19]	Russia	Prospective	32	24 months	NR	HSC	BM	MUD, haploidentical	16.5 months
Uppuluri *et al.* (2018)[36]	India	Retrospective	15	NA (under 18 years of age)	NA	HSC	BM, PSBC	MSC, MUD (not specified)	NA
Ferrua *et al.* (2019)[37]	Italy	Phase 1/2 clinical trial	8	26.4 months	III (2), IV (4), V (2)	HSC	BM & PBSC, CB	NA	43.2 months

(*Cont'd...*)

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Table 2. (*Continued*)

Author (Year)	Country	Study design	Sample size (*n*)	Mean age ± SD	WAS score	Cell type	Cell source	Donor	Follow-up duration
Iguchi *et al.* (2019)[38]	Japan	Retrospective	108	45.72 months	I (4), II (12), III (39), IV (34), V (15)	HSC	BM (62%), CB (30%), PBSC (8%)	35 RD, 69 UR, 4 N/A	103.44 months (2.14 –292.95 months)
Burroughs *et al.* (2020)[13]	USA	Retrospective	129	1.2 years (0.2–21.7 years)	I–II (53), III–IV (43), V (33)	HSC	BM (62%), PBSC (8%), CB (30%)	MSD (17%), MMRD (2%), MUD (32%), MMUD (16%), others (33%)	4.5 years (0.4–12.1 years)
Fernandes *et al.* (2019)[39]	Brazil	Retrospective	14	2 years (2 months to 19 years)	NR	HSC	BM & PBSC	Allogeneic (related donor)	2.4 years
Haskoloğlu *et al.* (2020)[40]	Türkiye	Retrospective	11	24 months (1–132 months)	III (1), IV (4), V (6)	HSC	BM & PBSC, CB	6 MSD, 3 MUD, 2 MMUD	8.5 years
Bildik *et al.* (2021)[41]	Türkiye	Retrospective	9	56.5 ± 50.8 months	V (5)	HSC	BM & PBSC, CB	HLA-matched relatives	9–182 months
Raj *et al.* (2021)[42]	India	Retrospective	35	12 months (1–220 months)	NR	HSC	PBSC (69.3%), BM (24%), CB (6.6%)	MSD, MUD, haploidentical	14.4 months (3.3–42.6 months)
Albert *et al.* (2022)[43]	Multi-city Europe	Retrospective	197	18 months	I–II (24), III–IV (71), V (40)	HSC	BM - CB	MSD: 39, MUD: 114, MMUD: 14, MMRD: 28	44.9 months (range, 25.5–70.4 months)
Magnani *et al.* (2022)[44]	France, UK	Phase 1/2 clinical trial	9	5.25 years (0.8 – 30 years)	III (1); V (7)	HSC GT	BM, PSBC	Autologous, GT	7.6 years

Abbreviations: BM: Bone marrow; CB: Cord blood; GT: Gene therapy; Haploidentical: Half-matched donor; HLA-ID: HLA-identical donor; HSC: Hematopoietic stem cells; HSPCs: Hematopoietic stem and progenitor cells; MMRD: Mismatched related donor; MMUD: Mismatched unrelated donor; MSD: Matched sibling donor; MUD: Matched unrelated donor; NA: Not available; Non-ID PD: Non-identical parental donor; NS: Not specified; PBSC: Peripheral blood stem cells; RD: Related donor; SAMUD: Single allele mismatched unrelated donor; UCB: Umbilical cord blood; URD: Unrelated donor.

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mortality of 13% (95% CI = 9.5%–16.5%) and moderate heterogeneity (I^2 = 54.53%) (Figure 2). Correspondingly, the pooled mean prevalence of overall survival was 87% ([95% CI = 83.5%–90.5%]; I^2 = 54.53%) (Figure S1). Three studies, including Yamaguchi *et al.*,[35] Ferrua *et al.*,[37] and Haskoloğlu *et al.*,[40] reported no deaths. Subgroup analysis showed significant heterogeneity only among high-risk-bias observational studies (I^2 = 65.76%). The most common causes of death were infection-related (n = 53/94; i.e., 64.2%), followed by GVHD (n = 26/76; i.e., 31.3%) (Figure S2 and S3). Other less commonly reported causes of death were intracranial hemorrhage, encephalitis, acute respiratory distress syndrome, and Addisonian crisis, among others, as reported in individual studies. Event-free survival was estimated at 84.6% across 12 studies including 394 patients (95% CI = 76.2%–93.0%), with considerable heterogeneity (I^2 = 84.39%) (Figure S4). Subgroup analysis showed heterogeneity attributable to high-risk observational studies (I^2 = 67.19%).

3.3.2. Acute and chronic graft-versus-host disease

The pooled prevalence of acute GVHD from 15 studies with data from 632 patients was 35.9% (95% CI = 31%–40.8%; I^2 = 27.74%) (Figure 3), with similar values in subgroups (33.7% and 39.4% in high-risk and low-risk observational studies, respectively). Heterogeneity was demonstrated among low-risk observational studies (I^2 = 72.54%). Meanwhile, chronic GVHD occurred in approximately 12% of participants post-HSCT, based on 13 studies involving 604 patients (95% CI = 7.9%–16.1%; I^2 = 50.3%) (Figure S5). Similarly, substantial heterogeneity was observed in low-risk bias observational studies (I^2 = 78.13%). Neither acute nor chronic GVHD occurred in studies of autologous GT HSCTs.[5,34,44]

3.3.3. Autoimmune manifestations

The pooled mean prevalence of autoimmune manifestations was 19% (95% CI = 12.5%–25.6%; I^2 = 82.15%) (Figure S6), based on data from 15 studies, pooling 723 patients. Observational studies with high-risk bias drove heterogeneity (I^2 = 86.46%), and other subgroups showed lower rates of autoimmune manifestations (11% and 10% in non-randomized controlled trials and low-risk-bias observational studies, respectively, compared with 26.1% in high-risk-bias observational studies). Autoimmune risk has also been reported in those receiving autologous transplantation.

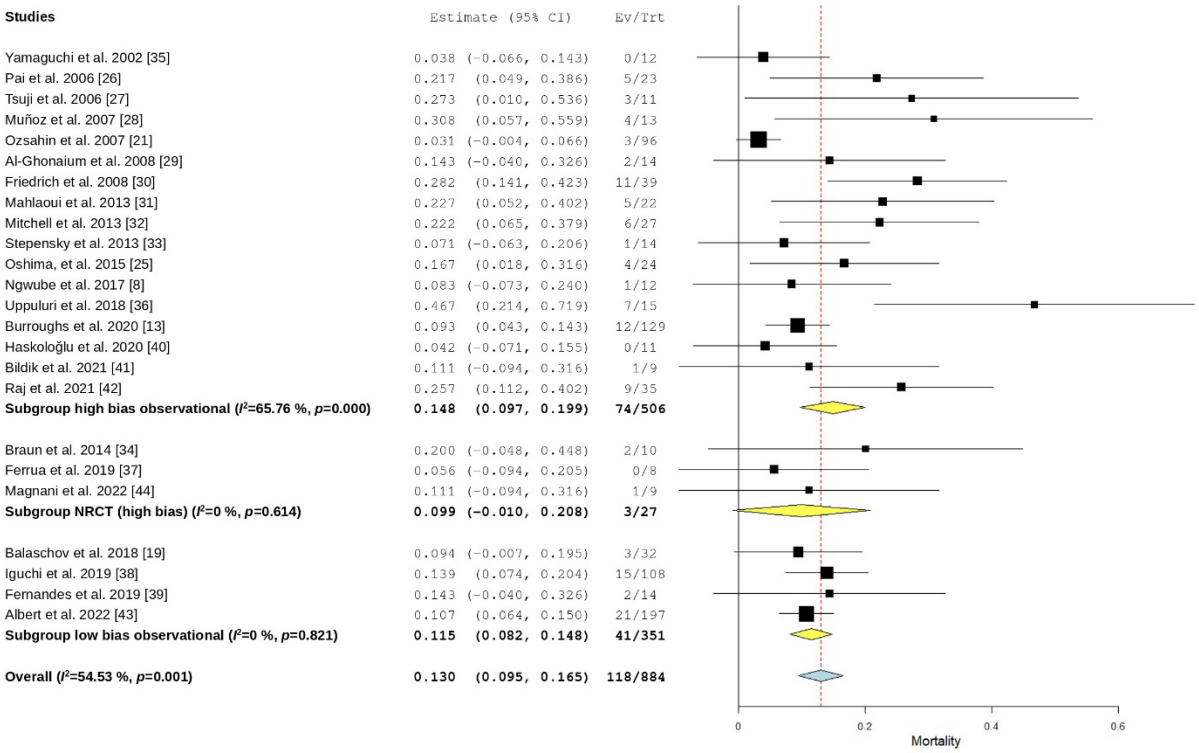


Figure 2. Forest plot showing the pooled prevalence of overall mortality across the included studies. The light-colored blue diamond shows the pooled estimate. The overall mortality rate was 13% (95% confidence interval: 9.5%–16.5%), based on 118 deaths in 884 of the total included patients.

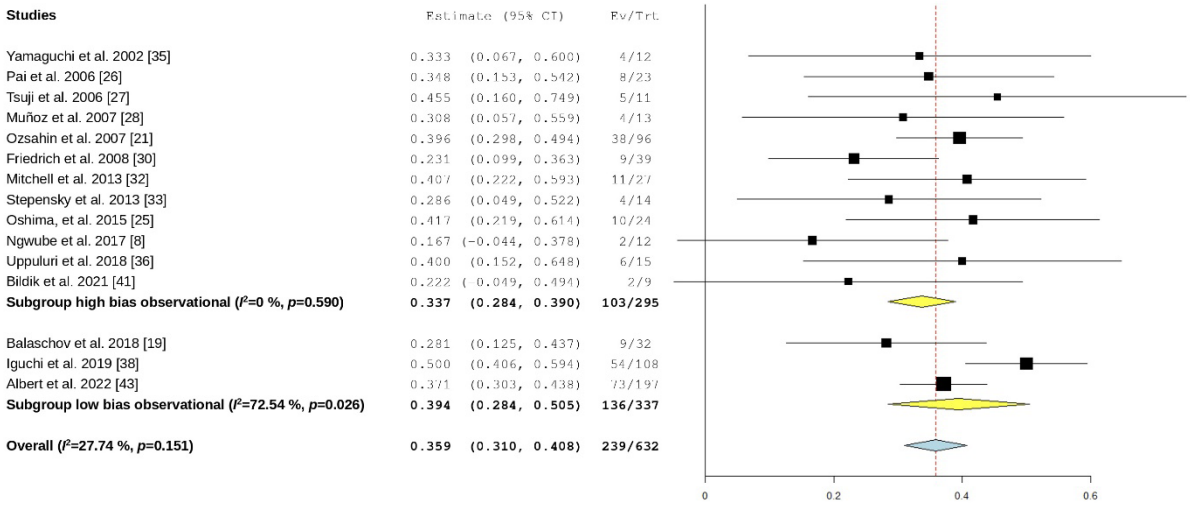


Figure 3. Forest plot of the pooled incidence of acute graft-versus-host disease (GVHD) in Wiskott–Aldrich syndrome patients after hematopoietic stem cell transplantation treatment. The pooled estimate shown by the light blue diamond is 35.9% (95% confidence interval: 31.0%–40.8%) with 239 GVHD events in 632 patients. The light blue diamond represents the pooled estimate, and the vertical red dashed line indicates the overall mean prevalence.

3.3.4. Symptomatic recovery

Among 13 reporting studies (n = 509 participants), 72% of patients achieved symptomatic recovery (72% [95% CI = 55%–88.9%]; I^2 = 94.89%) (Figure S7). Both low-risk and high-risk bias observational studies showed high heterogeneity (83.81% and 96.03%, respectively).

3.3.5. Platelet and neutrophil engraftment

Six studies reported the mean days to achieve neutrophil engraftment (Figure S8), with a pooled mean of 18.75 days post-HSCT (95% CI = 16.44–21.07 days; I^2 = 88.75%). Meanwhile, four studies reported a pooled mean day to platelet engraftment of 31.74 days post-HSCT (95% CI = 20.74–42.75 days; I^2 = 96.54%) (Figure S9). No subgroup analysis was possible for either outcome.

3.3.6. Full and mixed chimerism

Fifteen studies reported the post-transplantation chimerism rate, representing 676 patients. Full chimerism was achieved in 72.5% of cases (72.5% [95% CI = 64.7%–80.3%]; I^2 = 79.88%); subgroup analysis did not identify the source of heterogeneity, as both showed moderate heterogeneity (62.15%–74.45%) (Figure 4). Meanwhile, 18.9% of patients showed mixed chimerism (18.9% [95% CI = 12.2%–25.5%]). The source of heterogeneity was the low-risk bias in observational studies (I^2 = 90.41%) (Figure S10).

3.4. Quality of the studies

The NOS quality assessment applied to the observational studies is summarized in Table S1. Only four studies were considered good quality,[18,30,31,34] while the rest were poor quality (n = 17); almost all observational studies lost points in two main domains: selection and comparability. In contrast, we assessed the three non-randomized controlled trials using the ROBANS tool, which showed high-risk bias in all three non-randomized controlled trials (Table S2).

3.5. Sensitivity analyses

We performed a leave-one-out sensitivity analysis for each included outcome (i.e., mortality, GVHD, autoimmune manifestations, recovery, etc.) (Figures S11–S19). Across all outcomes, omitting an individual study did not statistically alter the pooled estimates, except for autoimmune manifestations. The leave-one-out sensitivity analysis revealed a significant change in the pooled proportion of autoimmune complications when omitting the study by Bildik *et al.*[41] (13.7% [95% CI = 9.7%–17.6%]).

4. Discussion

This systematic review and meta-analysis summarize the available evidence on clinical outcomes following HSCT in patients with WAS. The salient findings of our analysis of 24 eligible studies (i.e., 21 observational studies; 3 non-randomized controlled trials, with 884 participants) are:

(i) This meta-analysis specifically assessed the outcomes of HSCT in WAS patients (n=884 from a total of 24 studies, three of which were non-randomized controlled trials, thereby increasing the generalizability of this study).

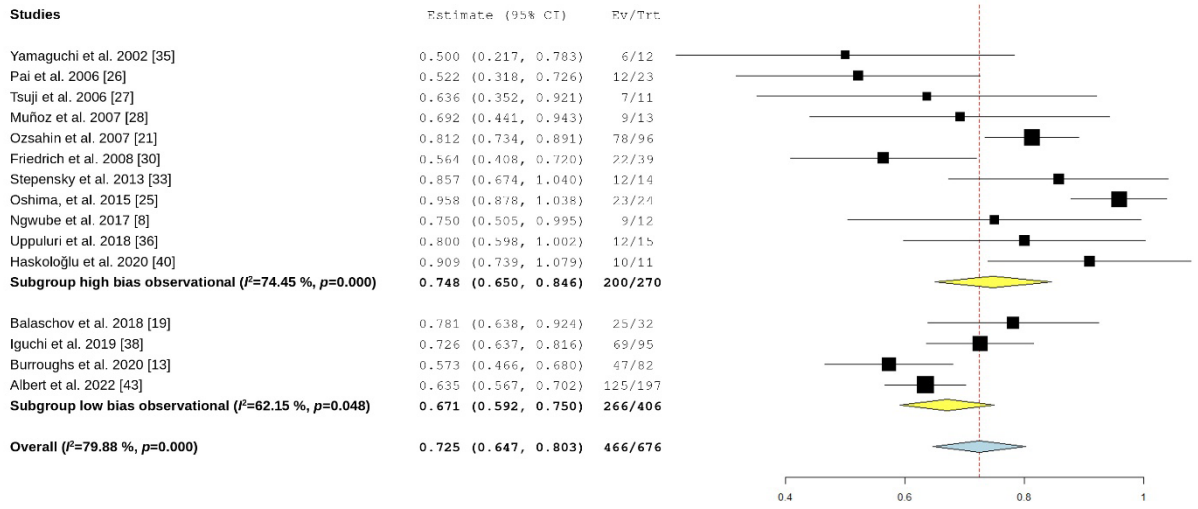


Figure 4. Forest plot of the pooled incidence of full chimerism in Wiskott–Aldrich syndrome patients after hematopoietic stem cell transplantation treatment. The pooled estimate shown by the light blue diamond is 72.5% (95% confidence interval: 64.7%–80.3%), with 466 of 676 patients achieving full chimerism. The light blue diamond represents the pooled estimate, and the vertical red dashed line indicates the overall mean prevalence.

(ii) There was an overall mortality of 13%, with more than half of reported deaths attributed to post-HSCT-related infections (63.6%), followed by GVHD (24.1%).
(iii) Overall survival was approximately 87%
(iv) Days to neutrophil and platelet engraftment were estimated at 18.75 and 31.74 days, respectively. Full chimerism was achieved in 72.5% of cases, whereas mixed chimerism occurred in 18.9% of patients. Among selected complications, the approximate rates of acute and chronic GVHD post-transplantation of 35.9% and 12%, respectively, were observed, and autoimmune complications occurred in 19% of patients post-HSCT.

The first successful implementation of HSCT in WAS dates back to 1968, in a two-year-old boy who received a bone marrow allograft from his sister.[46] This pioneering study was reported decades before Derry *et al.*[47] identified the *WASp* gene. Refinements in transplantation techniques and advancements in post-transplantation care have improved outcomes among HSCT recipients with WAS. More than half a century after Bach *et al.*[46] introduced HSCT,[46] our systematic review and meta-analysis sought a more holistic understanding of HSCT significance in patients with WAS, based on the available literature.

Various factors have been reported to influence HSCT outcomes in patients with WAS. Pre-transplant clinical status (i.e., lower clinical severity score, absence of pre-existing autoimmunity, severe infections), younger age at HSCT, HLA-matched sibling donors, and some conditioning regimens (e.g., busulfan-based regimens) were associated with better outcomes. Some experts have arbitrarily suggested a cutoff age of <5 years associated with better HSCT outcomes.[13,48–50] Albert *et al.*[51] found that the hazard ratio is more likely to increase with age. Furthermore, although mismatched donor status was historically considered to affect outcomes, more recent studies (i.e., since 2000) have found a smaller impact of donor type on overall survival and GVHD, which may reflect improvements in HLA matching, conditioning, and T-cell depletion strategies. Nevertheless, the gold-standard practice remains an HLA-identical sibling donor.[52] Regarding the conditioning regimen, some studies have reported higher disease-free survival and lower graft failure rates with a busulfan-based regimen than with other regimens.[33] Furthermore, the regimen showed better engraftment and a lower incidence of mixed donor chimerism compared with the treosulfan-based regimen. However, an interim analysis of a retrospective study by Albert *et al.*[51] found comparable cure rates of almost 90% between the two regimens among patients with WAS.

Our pooled data indicate a mortality rate of 13% among HSCT-recipient WAS patients, with infections remaining the main cause of death (63%). This is consistent with a meta-analysis of 12,581 deaths in inborn errors of immunity, which reported infections as the most prevalent cause of death post-HSCT.[53] In addition to immunosuppressive and conditioning regimens, pre-existing immunodeficiency, underlying phenotypes, pre-transplant infections, pre-existing organ damage, and multidrug resistance

colonization status in WAS patients may delay immune reconstitution following therapy, which may persist beyond two years post-HSCT.[54] Close follow-up of immunological aspects pre- and post-transplant was reported as an effective way to monitor infection burden through early diagnosis and intervention.[54] Furthermore, a major concern was related to post-transplant complications, particularly GVHD, which affected more than one-third of included patients. More recent studies suggest that certain conditioning regimens, i.e., busulfan or treosulfan, led to fewer GVHD events.[43] Although these conditioning protocols curtailed GVHD risk, reports suggested their association with toxicity.[55] Another strategy to reduce the risk of GVHD and the toxicity of conditioning drugs is the use of autologous HSCT, in addition to GT, in the treatment of WAS.

Despite the favorable outcomes observed, the study has several limitations. For example, we could not compare with appropriate control arms because most included studies described only HSCT recipients. This made the original PROSPERO protocol infeasible, so we adapted it to the available published data. Furthermore, most studies included in the meta-analysis were suboptimal (*n* = 17/21 observational studies) and, in most cases, lacked comparability because they were single-arm. We observed similar patterns in three non-randomized controlled trials, which may explain the high risk of bias. We excluded some non-English studies due to language restrictions. Subgroup analyses—i.e., analyses based on different covariates, such as baseline demographics, WAS severity score, pre-comorbid conditions, conditioning regimens, and others—were impractical, as many studies did not specify outcomes of individual patients. We believe including these data is imperative, as these factors directly contribute to outcomes. Another limitation is that comparisons between observational studies and randomized controlled trials are descriptively interpreted rather than as evidence of comparative efficacy. This may be because most studies did not report adjusted effect estimates. Hence, a pooled analysis based on adjusted outcomes was not feasible. Future studies should report adjusted effect estimates to enable more robust comparative analyses.

Future directions in HSCT for WAS should focus on the impact of the previously discussed variables on HSCT outcomes and on strategies to prevent post-transplantation complications, most importantly infections, GVHD, and related toxicities. Furthermore, integrating novel GT, particularly for patients without suitable donors, can improve therapeutic efficacy and reduce reliance on donors. These developments align with a growing trend toward more personalized treatments to improve survival and quality of life in patients with WAS.

5. Conclusion

Our data demonstrate that HSCT is associated with favorable survival and clinical outcomes in patients with WAS. We observed an overall survival rate of 87% and symptomatic recovery in nearly three-quarters of the HSCT-treated patients. However, these findings should be interpreted cautiously, as most included studies were single-arm and lacked a comparator group. Despite notable outcomes observed, the use of HSCT is not without its associated risks, encompassing cell therapy-related complications, including acute as well as chronic GVDH and autoimmune manifestations that may culminate in patient death (up to 13% mortality rates observed in our analysis). A key finding is the importance of intensive post-treatment supportive care in reducing recurrent infection among treated patients. Despite the favorable outcomes observed, the suboptimal quality of the published clinical studies included in the analysis warrants careful interpretation and calls for well-designed future studies and standardized outcome reporting, with longer-term follow-up to assess the sustainability of the observed clinical outcomes. Moreover, future studies should examine how variables such as patients' baseline status, conditioning regimens, and donor status affect outcomes and the quality of HSC preparation, as well as strategies to optimize post-transplantation status to prevent severe infections.

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Conflict of interest

The authors declare they have no conflicts of interest.

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Data are available from the corresponding author upon reasonable request.

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