

Real-world Clinical Outcomes of Autologous Stem Cell Transplantation in Chinese Patients with Newly Diagnosed Multiple Myeloma: A Systematic Literature Review

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BACKGROUND & OBJECTIVES

- Multiple myeloma (MM) is a common hematologic malignancy, primarily affecting older adults, and remains incurable despite therapeutic advances.
- Autologous stem cell transplantation (ASCT) significantly improves survival and offers deep, durable responses, but its use in China is low, especially among older patients.
- While newer therapies like proteasome inhibitors, immunomodulatory drugs, and Chimeric Antigen Receptor T-cell therapy are less invasive, they are costly and lack long-term real-world data.
- This study aimed to use real-world evidence to evaluate ASCT's clinical impact, describe its utilization in Chinese patients with newly diagnosed MM, and provide insights to guide future treatment decisions.

METHODS

Study Design

- Systematic literature review (SLR) following PRISMA guidelines
- Focused on real-world evidence of ASCT and non-ASCT treatments in Chinese patients with transplant-eligible newly diagnosed MM

Data Sources & Search Strategy

- Databases:** English (MEDLINE, Embase, Web of Science) Chinese (CNKI, WANFANG, VIP)
- Timeframe:** January 1, 2015, to January 15, 2025
- Keywords:** Combinations of terms relating to "Multiple Myeloma," "Newly Diagnosed," "China/Chinese," and "Real-World Evidence"

Study Selection

- Inclusion:** observational studies on ASCT/non-ASCT in transplant-eligible ndMM patients.
- Exclusion:** clinical trials, preclinical studies, case reports/series, non-human studies, reviews, guidelines, mixed/relapsed cohorts.

Data Extraction

- Captured:** study characteristics, patient demographics/staging/cytogenetics, treatment protocols, clinical outcomes (response, survival), and safety [adverse events (AEs), Common Terminology Criteria for Adverse Events grading].

Evidence Synthesis & Statistical Analysis

- Grouped studies by treatment phase (ASCT: induction, mobilization, engraftment, maintenance; non-ASCT: induction, maintenance).
- Single-arm meta-analysis (random-effects) and pooled Cox regression for overall survival (OS) where feasible.
- Cochran's Q test (continuous) and Chi-square tests (categorical) were used to compare treatment phases.
- Heterogeneity assessed via I²; significance defined as two-sided p < 0.05.

RESULTS

- The literature search process is illustrated in Figure 1. 88 references (37 English and 51 Chinese) met the predefined eligibility criteria and were retained for data extraction and evidence synthesis.

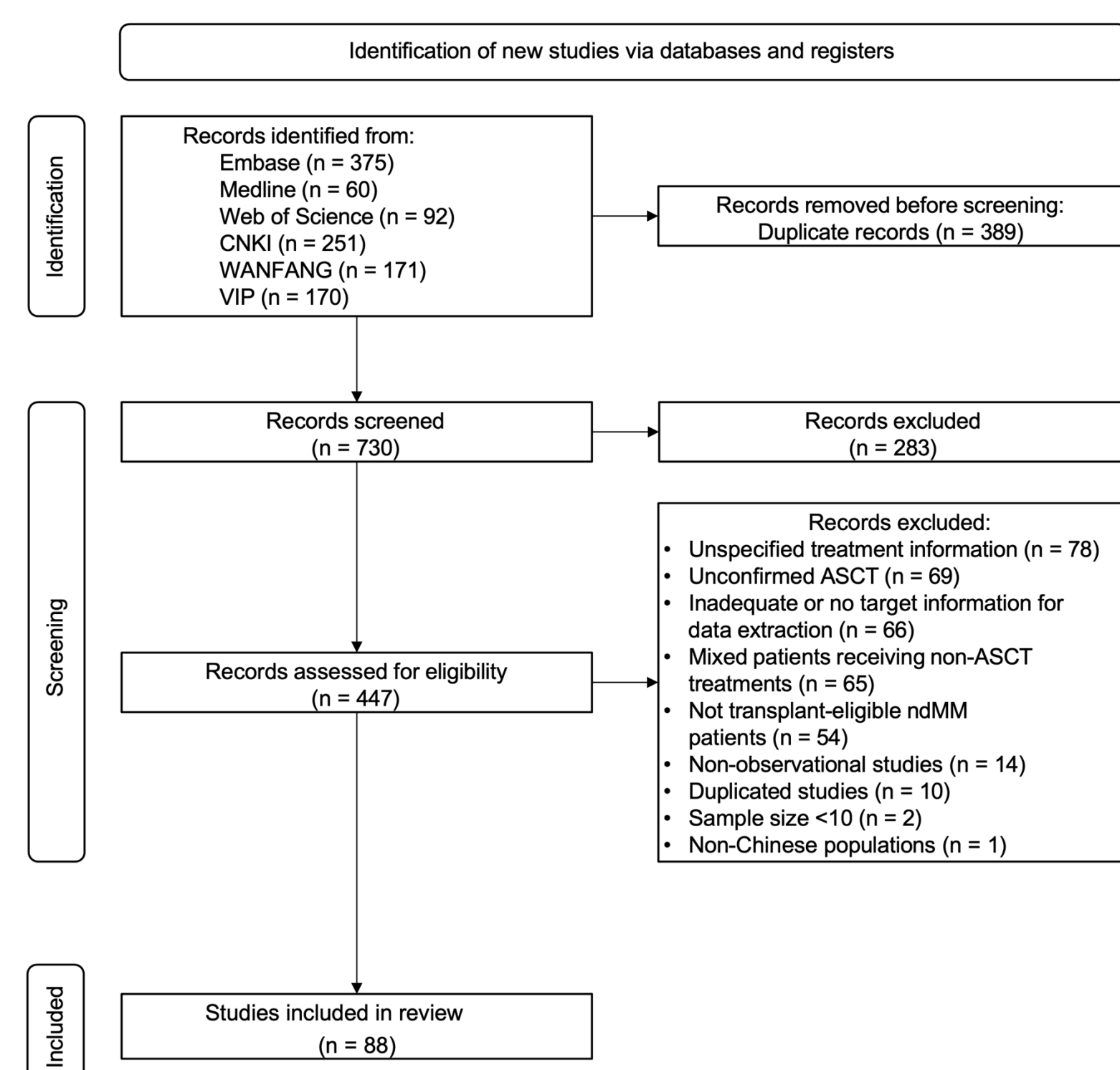


Figure 1. The flowchart of literature search

Patient characteristics

- The 29 included studies (10,785 patients) allowed comparison of baseline characteristics between ASCT (n=3,957) and non-ASCT (n=2,712) treatments. Detailed comparisons are presented in Table 1 and Figure 2.
- Patients selected for ASCT in the included Chinese studies were younger and more likely to present with favorable disease features, such as IgG monoclonal protein, ISS stage I disease, and t(11;14) translocation.
- This suggests a potential underutilization of ASCT among older adults in China and highlights the need to develop frailty-based rather than age-based selection tools in the Chinese setting.

Table 1. Pooled patient characteristics of Chinese patients receiving ASCT and non-ASCT treatments for transplant-eligible ndMM

Variable	ASCT group			Non-ASCT group			P value
	Number of studies	Sample size	Point estimation (95%CI)	Number of studies	Sample size	Point estimation (95%CI)	
Demographics							
Age	28	3,692	54.5 (53.2, 55.8)	18	2,566	61.4 (59.4, 63.4)	<0.001
Male gender	28	3,692	58.7% (56.3%, 61.0%)	18	2,566	56.4% (54.5%, 58.3%)	0.152
Monoclonal protein							
IgG	23	3,230	50.8% (49.1%, 52.6%)	14	2,108	44.9% (42.4%, 47.4%)	<0.001
IgA	23	3,230	21.1% (19.7%, 22.5%)	13	2,028	24.9% (22.4%, 27.5%)	0.008
Light chain	17	2,393	22.0% (19.7%, 24.3%)	12	1,992	22.3% (19.5%, 25.4%)	0.851
Kappa light chain	7	946	13.7% (4.8%, 33.5%)	1	140	47.9% (39.7%, 56.1%)	0.004
Lambda light chain	7	946	16.5% (8.4%, 29.8%)	1	140	49.3% (41.1%, 57.5%)	<0.001
Cytogenetics							
High risk	9	970	26.6% (15.3%, 42.1%)	6	1,046	24.3% (15.5%, 36.0%)	0.787
t(4; 14)	8	1,917	13.4% (9.5%, 18.6%)	3	722	10.4% (7.2%, 14.8%)	0.306
del(17p)	7	1,439	14.2% (12.5%, 16.1%)	1	268	12.3% (8.9%, 16.8%)	0.402
Standard risk	7	413	45.0% (31.1%, 59.8%)	3	475	45.7% (21.8%, 71.9%)	0.965
t(11; 14)	7	1,840	14.1% (12.6%, 15.8%)	1	335	6.0% (3.9%, 9.1%)	<0.001
Comorbidities							
Extramedullary diseases	5	582	21.3% (16.8%, 26.7%)	4	681	18.4% (12.8%, 25.9%)	0.499
Hypertension	1	93	32.3% (23.6%, 42.4%)	3	481	38.5% (33.0%, 44.3%)	0.283
Diabetes	1	93	18.3% (11.7%, 27.5%)	2	382	10.8% (6.7%, 16.9%)	0.102

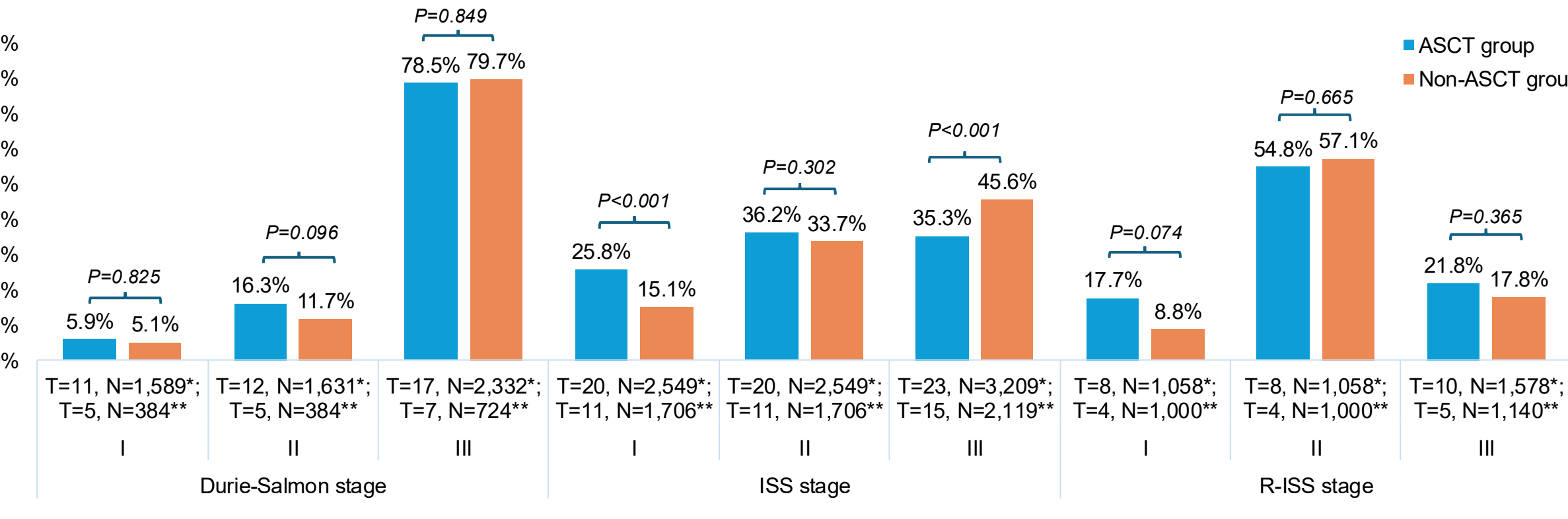


Figure 2. Disease staging of Chinese patients receiving ASCT and non-ASCT treatments for transplant-eligible ndMM

Outcomes of mobilization, engraftment, and hematopoietic recovery in the ASCT receipts

- Three studies (n=212) evaluated plerixafor + G-CSF and six studies (n=419) assessed cyclophosphamide + G-CSF for stem cell mobilization.
- With respect to mobilization and engraftment, our study demonstrated that plerixafor plus G-CSF was more effective than cyclophosphamide plus G-CSF in achieving successful stem cell mobilization (73.6% vs. 49.5%, P = 0.001).
- PXF + G-CSF mobilized significantly higher yields of CD34+ and mononuclear cells compared to the Cy-based regimen, while achieving comparable granulocyte and platelet engraftment times (Figure 3).

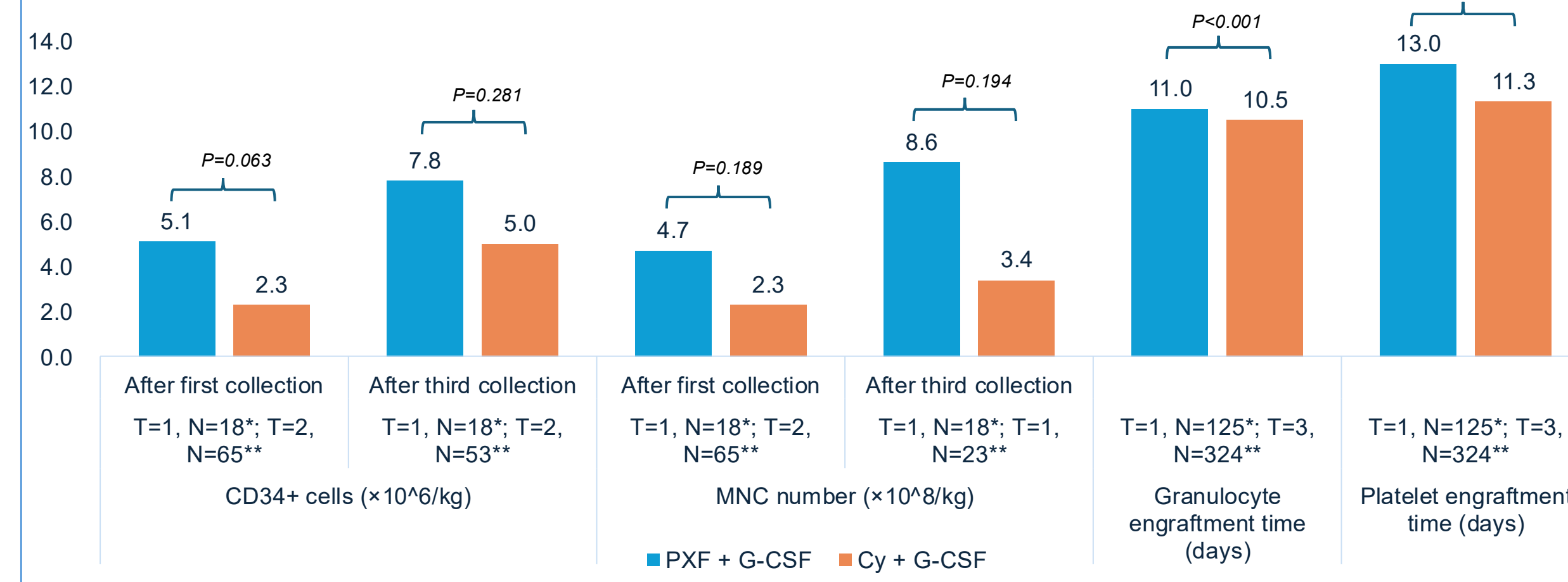


Figure 3. Comparisons of pooled engraftment outcomes between two mobilization regimens in Chinese patients receiving ASCT for ndMM

Treatment response and survival outcomes

- Pooled analyses showed significantly better treatment responses with ASCT compared to non-ASCT treatments, with higher sCR/CR rates (78.5% vs. 45.3%) and ≥VGPR rates (98.6% vs. 75.0%, P=0.011) (Figure 4).
- ASCT was also associated with superior survival outcomes, including higher 5-year OS (70.6% vs. 41.3%, P<0.001) and 5-year PFS (42.0% vs. 18.4%, P<0.001).

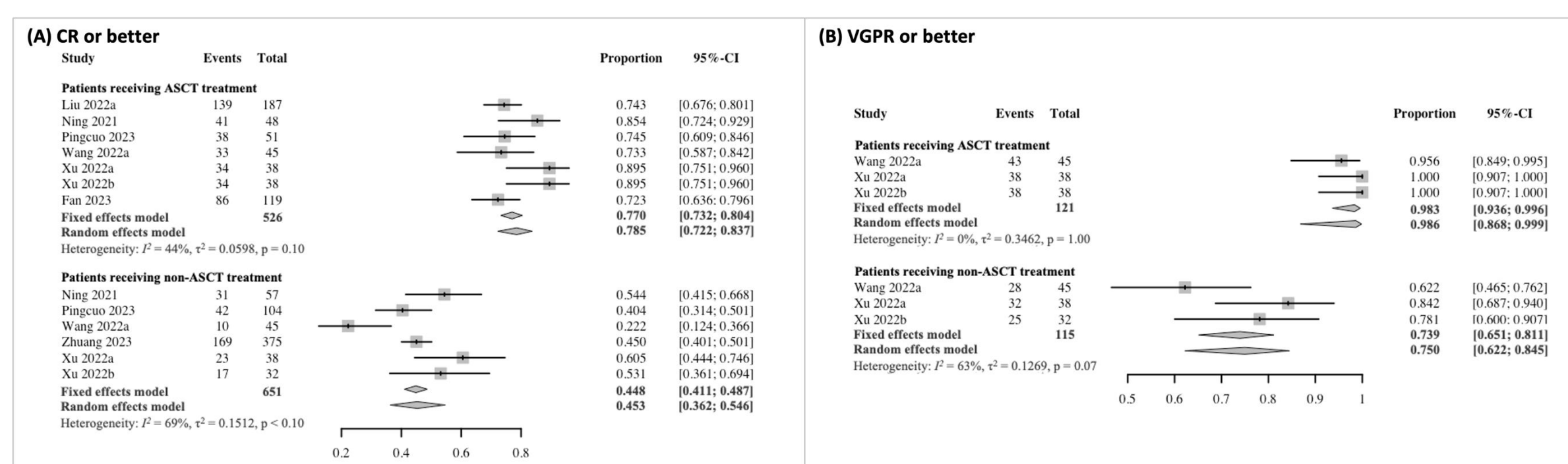


Figure 4. Comparison of pooled treatment response outcomes between ASCT and non-ASCT treatments in Chinese patients with transplant-eligible ndMM

- Subgroup analyses suggested improved survival with PI+IMiD induction followed by IMiD maintenance and better PFS with PI-based versus IMiD-only induction (Figure 5).

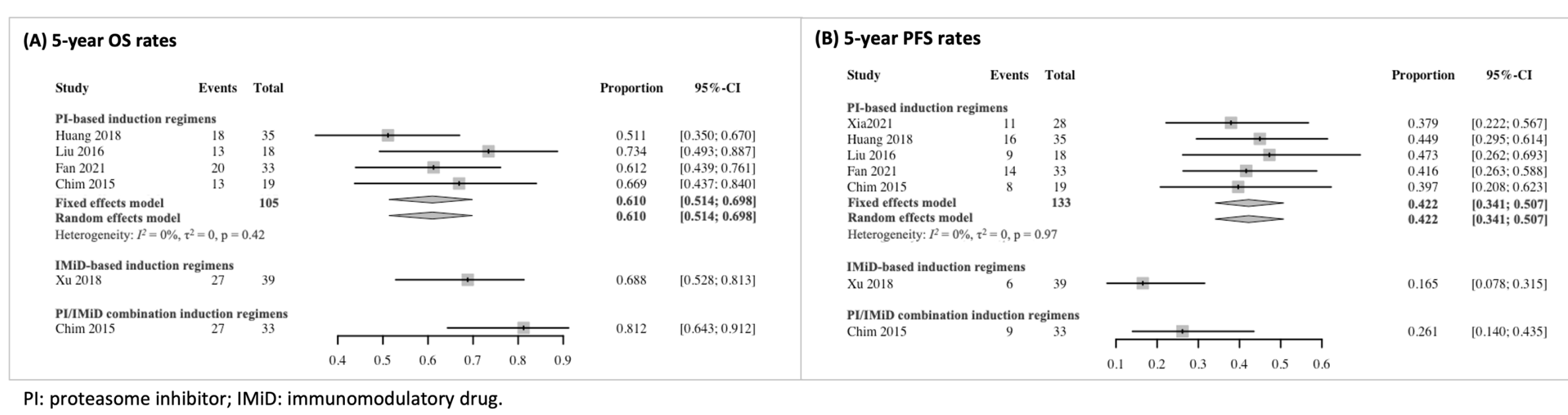


Figure 5. Comparisons of the pooled survival outcomes between induction treatments followed by IMiD maintenance in Chinese patients receiving ASCT for ndMM

Factors associated with Survival outcomes in the ASCT receipts

- Cox regression analyses identified several prognostic factors for survival after ASCT (Figure 6).
- Advanced ISS/R-ISS stage and early relapse (<12 months) were associated with significantly higher mortality risk (pooled HR up to 18.52), while MRD negativity and achieving ≥VGPR post-ASCT predicted better survival outcomes.
- Similarly, advanced disease stage, IgD subtype, and suboptimal response before ASCT predicted poorer PFS, whereas MRD negativity was associated with reduced progression risk.

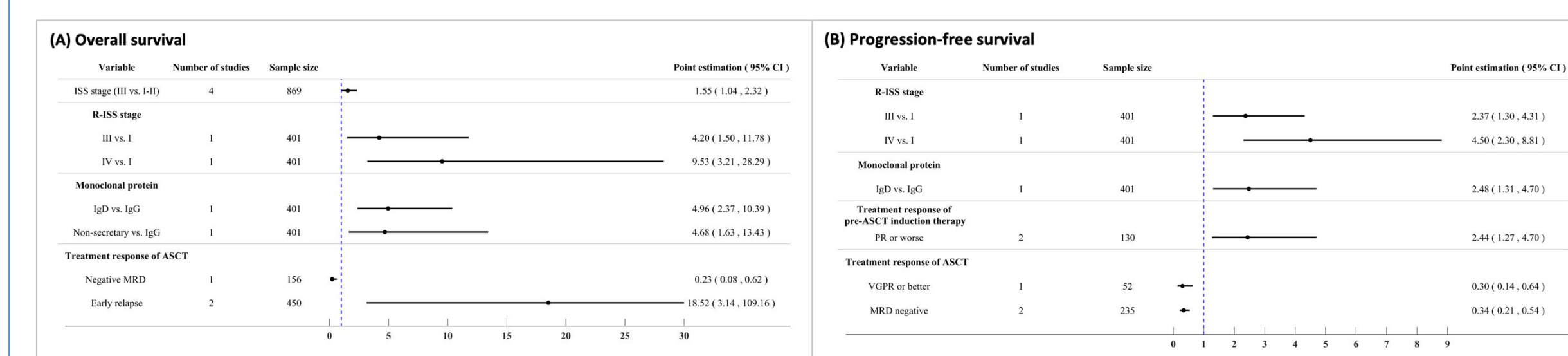


Figure 6. Pooled HR of prognostic factors for survival outcomes in Chinese patients receiving ASCT for ndMM

Severe AE related to ASCT

- Pooled safety analysis showed that severe (Grade ≥3) adverse events after ASCT were relatively infrequent, with diarrhea (11.8%) and infection (7.6%) being the most common.
- Other notable high-grade adverse events included gastrointestinal toxicity (4.8%), mucositis (~5%), and hepatic toxicity (3.9%).

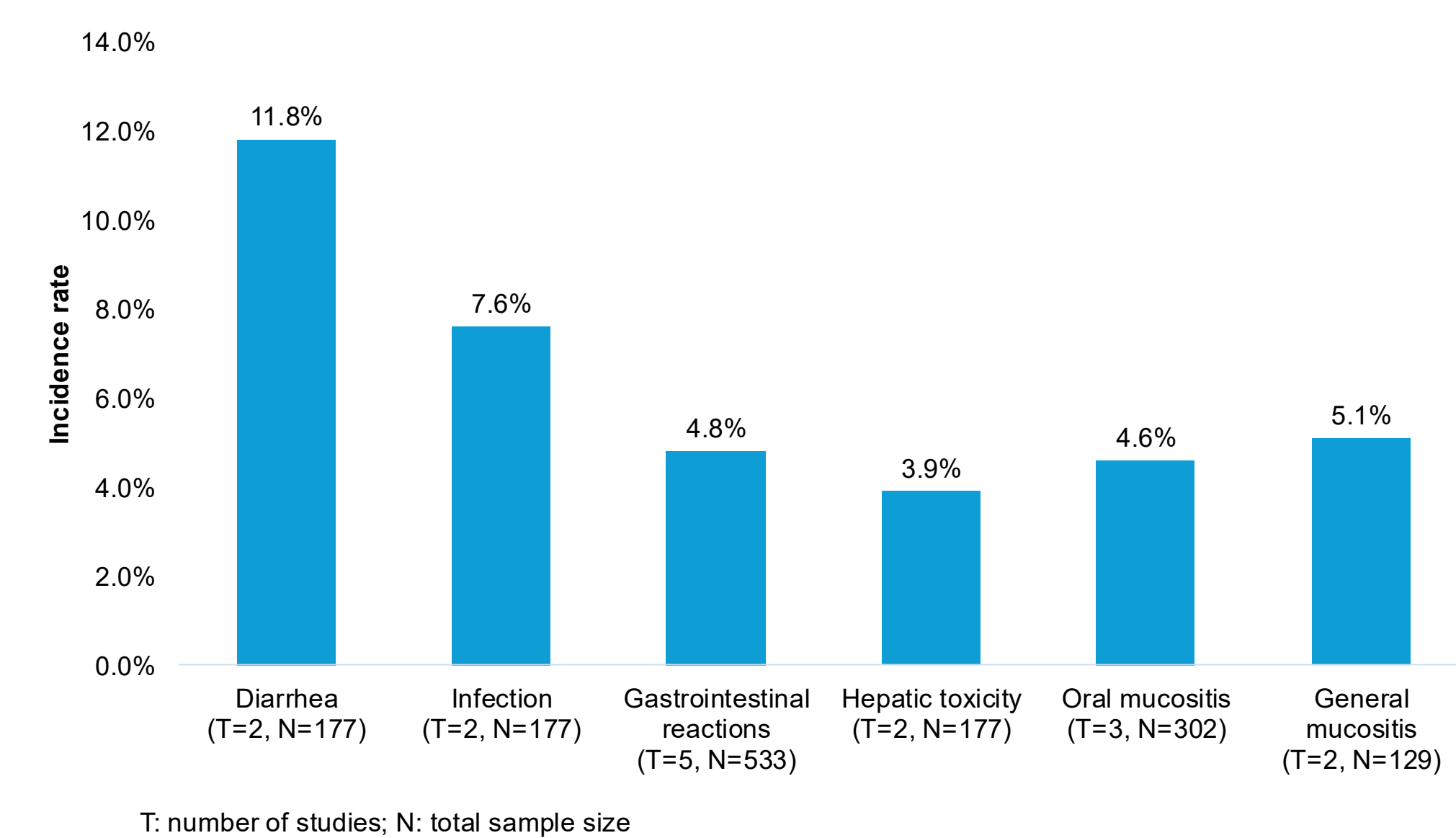


Figure 7. Severe AE related to ASCT

CONCLUSIONS

- This SLR confirms that ASCT remains an effective and safe standard of care for transplant-eligible Chinese patients with ndMM, achieving high response rates and favorable survival outcomes.
- Real-world evidence suggests superior outcomes with ASCT compared to non-ASCT treatments, particularly when combined with modern induction and maintenance strategies.
- Further real-world and cost-effectiveness studies are needed to confirm these advantages and support clinical and reimbursement decisions.