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Survival Differences in Transplant Ineligible Multiple Myeloma Patients in Japan and Taiwan: Exploring the Potential Influence of Timely and Unrestricted Access to Novel Therapies

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Key Takeaway

- Timely and unrestricted access to novel therapies, for example, daratumumab, significantly improves survival outcomes and disease control in patients with TIE-NDMM.
- Sustained access is critical for maintaining disease control. In Taiwan, reimbursement of new treatments is initially restricted to the relapsed/refractory setting with limitations on funding (e.g., 22 infusions for daratumumab). This may prevent patients from staying on treatment long enough to achieve optimal outcomes, unlike Japan where access is granted in the newly-diagnosed setting and is unrestricted.
- After adjusting for age, sex, and comorbidities, Japanese patients had a hazard ratio of 0.24 for death and lived on average 14.6 months longer over 4 years compared to Taiwanese patients.

Conclusions

- Earlier and sustained access to innovative therapies like daratumumab is associated with significantly better survival in TIE-MM. These findings highlight a need for policy changes to improve access to novel treatments and reduce regional disparities in outcomes.

Congress Hub Presentation: https://www.congresshub.com/ASH2025/Oncology/Daratumumab/Hou

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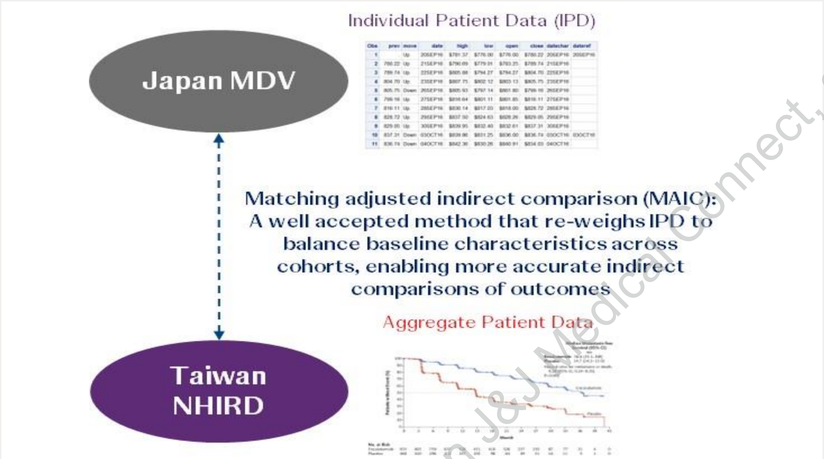
Introduction

- Novel therapies such as daratumumab have significantly improved survival outcomes in transplant-ineligible (TIE) multiple myeloma (MM).[1] However, timing and scope of access and reimbursement vary widely across countries, impacting real-world patient outcomes.
- Japan and Taiwan both approved daratumumab for use in first line for TIE MM in 2019; however, it is only reimbursed in the frontline setting in Japan.
- By contrast, Taiwan limits reimbursement of daratumumab to relapsed/refractory settings, with reimbursement approved only in 2020 with capped usage of 22 infusions.
- This analysis compares overall survival (OS) in patients with TIE newly diagnosed multiple myeloma (NDMM) between Japan and Taiwan to highlight the value of timely and unrestricted access of daratumumab.

Objective: To conduct a matching-adjusted indirect treatment comparison (MAIC)[2] to evaluate the impact of access timing and reimbursement on the OS of patients with TIE-NDMM in Japan and Taiwan.



MDV, Medical Data Vision; NHIRD, National Health Insurance Research Database

Results

Study population

- Prior to MAIC weighting, Japanese patients were older (mean age: 74.2 vs. 71.2 years) with a higher comorbidity burden (mean CCI: 5.36 vs. 3.23) Taiwanese patients (Table 1).
- After MAIC re-weighting, Japan and Taiwan cohorts were well-balanced on age, sex, and comorbidities (CCI scores) (Table 1).

Table 1: MAIC baseline matching results

Variable	Taiwan N=446	Japan N=445	Re-weighted Japan ESS=244
Mean age (y)	71.2	74.2	71.2
Male (%)	51.3	51.5	51.3
Mean CCI score	3.23	5.36	3.23
CCI Score = low (%)	6.05	1.80	6.05
CCI Score = medium (%)	37.2	15.3	37.2
CCI Score = high (%)	36.1	28.8	36.1
CCI Score = very high (%)	20.6	54.2	20.6

ESS, effective sample size; CCI, Charlson Comorbidity Index

Methods

Data sources

Nationally representative real-world patient-level data from Medical Data Vision database, Japan, and summary-level data from the National Health Insurance Research Database, Taiwan.

Patients

- Eligible adults with TIE-NDMM diagnosed between 1 January–31 December 2020 who had received first-line therapy. Patients receiving autologous stem cell transplant or with other primary cancers were excluded. Patients were followed until death or 31 December 2023.

Analysis

- A harmonized set of eligibility criteria were applied across both cohorts. An unanchored Bayesian and Frequentist MAIC was conducted using nationally representative datasets along with quantitative bias analysis (QBA). The Japan cohort was re-weighted using clinically relevant covariates (age, sex, Charlson Comorbidity Index [CCI]) from the Taiwan cohort (N=446) were used to reweight the Japan cohort (N=445).
- Both 95% confidence (and credible) intervals were calculated. OS was the primary outcome.
- Survival was expressed as restricted mean survival time (RMST), a measure of treatment effect presented as a gain or loss in event-free days (event-free-survival).

MAIC-adjusted survival

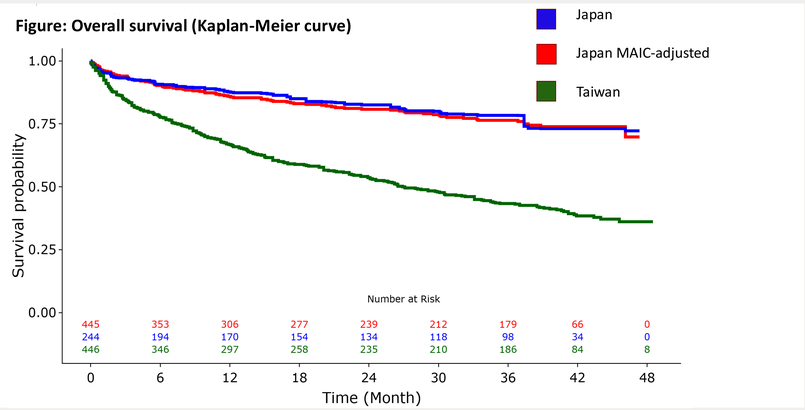
- MAIC-adjusted results showed significantly better overall survival for Japan vs. Taiwan patients (Table 2)
- Frequentist HR: 0.24 (95% CI: 0.177–0.327)
- Bayesian HR: 0.2403 (95% CrI: 0.1734–0.3262)
- At 12, 24, 36, and 45 months, the MAIC-adjusted OS probabilities in Japan were 88.6%, 86.0%, 83.7%, and 80.7%, respectively compared to 66.8%, 53.6%, 43.3%, and 37.2% in Taiwan.
- Median OS was not reached in Japan after approximately 4 years, compared to 26.9 months in Taiwan (Figure). The mean survival difference (RMST) was 14.6 months (95% CI: 11.9–17.3) over 4 years.
- QBA showed the results were robust to unmeasured confounders with an E-value of 4.7.

Table 2: Comparison of overall survival Taiwan versus Japan - MAIC

Results	Unadjusted values N=445	Adjusted values N=244
Frequentist analysis		
HR (95% CI); p-value	0.339 (0.267,0.43) <0.001	0.24 (0.177,0.327) <0.001
Restricted mean survival time difference (months) (95% CI) at month 48); p-value	12.3 (9.91,14.7) <0.001	14.6 (11.9,17.3) <0.001
Bayesian analysis		
HR (95% CrI)	0.3391 (0.2648,0.4287)	0.2403 (0.1734,0.3262)

CI, confidence interval; CrI, credible interval; HR, hazard ratio

Figure: Overall survival (Kaplan-Meier curve)



Conclusion and limitations

- Japanese patients had significantly longer OS both pre and post MAIC adjustment, despite an average older age and higher comorbidity burden.
- Study limitations include potential unmeasured confounders, eg, disease-specific characteristics such as stage at diagnosis, and differences in clinical practice beyond treatment.
- Despite this, the observed difference in unadjusted and adjusted survival outcomes between Japanese and Taiwanese patients may be partially explained by differential access to innovative therapies.

References

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Multiple Myeloma

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