Real-World Inpatient Outcomes Associated With Caplacizumab Use in Immune Thrombotic Thrombocytopenic Purpura

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INTRODUCTION

- Caplacizumab improves early disease control in immune thrombotic thrombocytopenic purpura (iTTP).
- However, real-world inpatient outcomes following its widespread adoption remain incompletely characterized.

AIM

To evaluate mortality, thrombotic and bleeding events, length of stay (LOS), and resource utilization among adults hospitalized with iTTP who received plasmapheresis (PEX), caplacizumab, or both.

METHOD

- **Study Design:** Retrospective cohort study using the National Inpatient Sample (2020–2023).
- **Cohorts:** Patients were grouped into three treatments: Caplacizumab + PEX, PEX without Caplacizumab (PEX-only reference group), and Caplacizumab without PEX.
- **Analysis:** Survey-weighted multivariable logistic and linear regression models adjusted for age, sex, rituximab use, and comorbidity indices.

CONCLUSIONS

- Among iTTP hospitalizations, caplacizumab plus PEX was not associated with significantly lower adjusted inpatient mortality compared with PEX alone.
- Caplacizumab-treated hospitalizations were associated with longer LOS and higher hospital charges.
- These findings should be interpreted cautiously because of the potential for confounding by indication and differences in illness severity that cannot be fully captured in administrative data.

RESULTS

- The analysis included 1,653 unweighted hospitalizations, representing an estimated 8,265 national admissions.
- **Cohort Sample Sizes (Weighted):** Caplacizumab + PEX (N=575); PEX without Caplacizumab (N=5,985); Caplacizumab without PEX (N=1,705).
- **Adjusted Outcomes:** Caplacizumab + PEX was not significantly associated with lower mortality compared to PEX alone (aOR 0.68; 95% CI, 0.32–1.45) or with the primary composite outcome (aOR 1.06; 95% CI, 0.68–1.65).
- **Resource Utilization:** Caplacizumab use with or without PEX was associated with significantly longer LOS (3.4 and 5.4 additional days, respectively; P<0.01) and higher total charges (P<0.01).

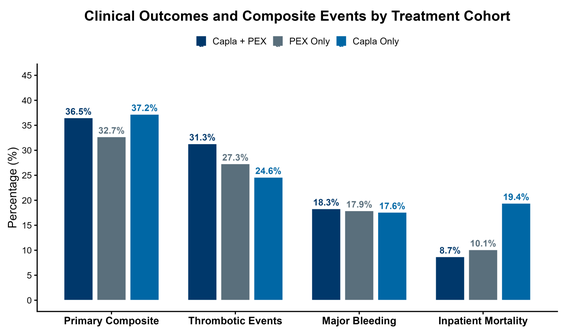

Figure 1. Unadjusted event rates by iTTP treatment cohort. Major bleeding was consistent across all groups, whereas inpatient mortality was highest for caplacizumab without PEX.

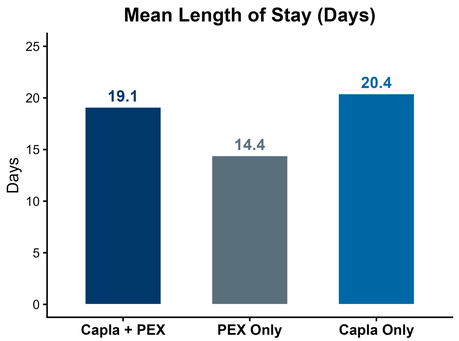

Figure 2. Mean length of stay (LOS) in days per iTTP hospitalization. Admissions utilizing Caplacizumab, both with and without PEX, demonstrated significantly prolonged hospital stays compared to the historical PEX-only control group (P<0.01).

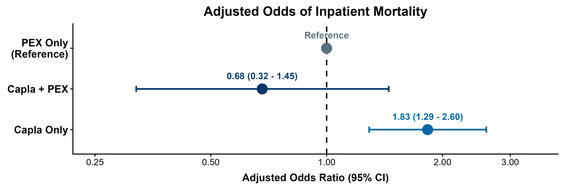

Figure 2. Adjusted odds ratios for inpatient mortality. Caplacizumab use without PEX was associated with significantly increased odds of mortality. Conversely, Caplacizumab combined with PEX did not demonstrate a statistically significant mortality reduction compared to the PEX-only reference group.

LIMITATIONS

- Administrative database research is subject to potential confounding by indication and uncaptured differences in baseline illness severity.
- Outcomes in the Caplacizumab without PEX cohort must be interpreted cautiously, as this group may be heavily confounded by coding artifacts or the inclusion of high-risk patients transitioned to comfort care before PEX initiation.

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