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Clinical Benefits of Caplacizumab: Findings from Real-World Data Analysis of Patients with Immune-Mediated Thrombotic Thrombocytopenic Purpura in Italy

This infographic is intended for researchers and healthcare professionals and reflects the contents of the following article:

Angelucci, E., Artoni, A., Fianchi, L., Dovizio, M., Iacolare, B., Saragoni, S., & Esposti, L. D. (2024). Real-World Data Analysis of Patients Affected by Immune-Mediated Thrombotic Thrombocytopenic Purpura in Italy. *Journal of Clinical Medicine*, *13*(5), 1342. <u>https://doi.org/10.3390/jcm13051342</u>

Thrombotic thrombocytopenic purpura (TTP)

TTP, a rare blood disorder, is characterised by the formation of blood clots in small blood vessels, leading to low platelet counts and anaemia

Pathogenesis of TTP

- Can be congenital (cTTP) or immune-mediated (iTTP)
- Deficiency of a disintegrin and metalloproteinase with a thrombospondin type 1 motif member 13 (ADAMTS13) that cleaves von Willebrand factor (VWF) causes TTP
- Impaired ADAMTS13 activity results in the accumulation of VWF multimers on the endothelial surface
- Subsequently, platelet aggregation and the formation of blood clots (thrombi) are triggered
- This cascade of events ultimately results in end-organ ischaemia and damage
- Most affected organs include the central nervous system, heart, and kidneys

Management of TTP





- Traditional therapeutic approaches include:
- Therapeutic plasma exchange
- Immunosuppressive therapy



'Exacerbation' or the worsening of TTP due to the persistent antibody-mediated ADAMTS13 deficiency is common in patients with iTTP



Caplacizumab is a novel anti-VWF, humanised, single-variable domain immunoglobulin



Caplacizumab

It inhibits the interaction between VWF multimers and platelets, thereby preventing the formation of blood clots



The effectiveness of caplacizumab has been studied in two clinical trials, TITAN (2016) and HERCULES (2019)

Corroboration of the efficacy and safety of caplacizumab with real-world evidence studies in Italy is lacking

Real-world analysis to investigate the epidemiology, demographic, and clinical characteristics of patients with iTTP in Italy before and after the introduction of caplacizumab



An observational, retrospective analysis using data retrieved from the administrative databases of Italian healthcare entities

Study participants

- Hospitalised adult (≥ 18 years) patients with a diagnosis of iTTP
- Patients with iTTP (N = 393)
- Patients with iTTP who received caplacizumab (N = 42)
- Patients with iTTP who did not receive caplacizumab (N = 351)



Data was extracted from the following databases:

- Demographic database
- Pharmaceuticals database
- Hospitalisation database
- Outpatient specialist services database
- Exemption database



Statistical analyses

Descriptive methods were utilised to analyse the treatment outcomes, ICU hospitalisations, and mortality rates associated with caplacizumab



- Propensity score matching (PSM) at 1:4 for matching the baseline characteristics of patients who received caplacizumab with caplacizumab-untreated patients
- Baseline characteristics of caplacizumab-untreated patients with iTTP after PSM-balancing for age and Charlson Comorbidity Index
 - Caplacizumab-untreated patients (N = 168)
 - $\circ~$ Mean (± SD) number of previous iTTP hospitalisations per patient: 0.7 \pm 2.0
 - Mean (± SD) duration of previous iTTP hospitalisations: 53.2 ± 73.6 days

Benefits of caplacizumab treatment for patients with iTTP

- No deaths after 1 month and 3 months from the first prescription of caplacizumab
- Mean duration of hospital stay in the ordinary setting (for any cause with 1-year follow-up): 15.7 ± 11.1 days
- ICU admissions during the first year of follow-up: <9.5% (N \leq 4)
- Overall duration of the ICU hospitalisations: <4 days
- Mean number of caplacizumab vials prescribed during the entire follow-up period (13.5 months): 31.2 ± 20.3 per patient

Hospitalisations and mortality in the unadjusted cohort of untreated patients (N = 351)



Key messages

- The real-world data analysis showed that caplacizumab therapy improves both clinical and patient-related outcomes in patients with iTTP
- Caplacizumab is associated with lower mortality rates, fewer ICU admissions, and shorter hospital stays, enhancing the overall quality of life for patients with iTTP



Real-World Data Analysis of Patients Affected by Immune-Mediated Thrombotic Thrombocytopenic Purpura in Italy Angelucci *et al.* (2024) | *Journal of Clinical Medicine* | DOI: 10.3390/jcm13051342

