

Real world data on Caplacizumab: our on-going experience

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Acquired thrombotic thrombocytopenic purpura (aTTP) is a rare life-threatening condition. Recently, Caplacizumab was approved for the treatment of acute episode of aTTP added to plasma exchange (PEX) and immunosuppresses at least for 30 days after withdrawal of daily PEX. Caplacizumab inhibits platelet adhesion to VWF multimers preventing micro vascular thrombosis. We report our experience with Caplacizumab (collected data September 2020-December 2021) in ten patients admitted in our Internal Medicine Department, diagnosed with aTTP. Patients' cohort baseline characteristics were reported in Tab.1. Neurologic symptoms occurred in all patients (focal, transient ischemic attack, drowsiness). The patients were submitted to daily PEX as administered steroids and caplacizumab. Median time to platelet count normalization (4 days), duration of PEX (8 days), and hospital stay (13 days) were comparable with RCT data, with complete neurological symptoms remission in all patients, in absence of haemorrhagic adverse events or death. Immunosuppressive therapy in aTTP aims to control the underlying autoimmune disease, but requires time to take effect, leading patients to thrombotic complications and death. Caplacizumab treatment prevents disease exacerbations, death and long-term sequelae, irrespective of the type of initial immunosuppression used, allowing time for immunosuppressive therapy to take effect, reducing length of hospitalization.

Age, mean (range), y	51 (30-82)
Female sex, % (n/total)	60 (6/10)
Platelets, initial, median (range), $\times 10^9/L$	18 (15-22)
LDH, initial, median (range), U/L	525 (380-698)
ADAMTS13 activity below 10%, % (n/total)	100 (10/10)
Glasgow Coma Scale, % (n/total)	
< 13	20 (2/10)
13-15	80 (8/10)

Table 1. Patients' cohort baseline characteristics were reported

References

1. Peyvandi F, Scully M, Kremer Hovinga JA (2016) TITAN Investigators. Caplacizumab for acquired thrombotic thrombocytopenic purpura. *N Engl J Med* 374:511-522.
2. Scully M, Cataland SR, Peyvandi F (2019) Hercules Investigators Caplacizumab treatment for acquired thrombotic thrombocytopenic purpura. *N Engl J Med* 380:335-346.