

differences on efficacy and safety outcomes that may have been undetected in the individual trials. **Methodology:** In both trials, patients with an acute episode of aTTP were randomized to receive CPLZ or placebo (PBO) in addition to therapeutic plasma exchange (TPE) and immunosuppression. All randomized patients from both studies were included in the integrated efficacy analyses (CPLZ: n=108; PBO: n=112), and those who received at least 1 dose of the study drug were included in the safety analyses (CPLZ: n=106; PBO: n=110). **Results:** CPLZ significantly reduced mortality (0 vs 4 deaths; $P<0.05$) and refractory TTP (0 vs 8 events; $P<0.05$) versus PBO and improved time to platelet count response (hazard ratio, 1.65; $P<0.001$). CPLZ also reduced the composite endpoint of TTP-related death, exacerbation, or any treatment-emergent major thromboembolic event during the treatment period (13.0% vs 47.3%; $P<0.001$) and median number of TPE days (5.0 vs 7.5 days) versus PBO. Mild mucocutaneous bleeding was the main safety finding for CPLZ. **Conclusion:** This integrated analysis provided new evidence that CPLZ prevents mortality and refractory disease in aTTP and reinforced the individual trial efficacy and safety findings. No new safety signals were identified for

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EPIDEMIOLOGY, TREATMENT PATTERNS, AND CLINICAL OUTCOMES AMONG PATIENTS WITH ACQUIRED THROMBOTIC THROMBOCYTOPENIC PURPURA (aTTP) IN THE UNITED STATES: AN ELECTRONIC HEALTH RECORDS ANALYSIS

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Objective: Acquired thrombotic thrombocytopenic purpura (aTTP) is an ultra-rare, potentially life-threatening thrombotic microangiopathy (TMA). Data on epidemiology, disease management, and clinical outcomes are scarce and often heterogeneous. The aim of this study was to assess the epidemiology, disease management, and clinical outcomes in patients with aTTP in the United States. **Methodology:** This longitudinal retrospective observational study of the Optum-Humedica database included patients with aTTP diagnosis from October 2015 to December 2019 if they had ≥ 1 documented ADAMTS13 activity $<10\%$ or ≥ 1 aTTP episode (≥ 1 inpatient stay with TMA diagnosis and ≥ 1 therapeutic plasma exchange [TPE] during the same stay); patients with conditions that mimic aTTP were excluded. Patients were followed until loss to follow-up, end of study period, or death. All analyses were descriptive. **Results:** Among 666 patients with aTTP diagnosis, 302 (45%) had ≥ 1 aTTP episode. Annual incidence of ≥ 1 aTTP episode was 1.81/million (based on data from 2016–2019). Patients with ≥ 1 aTTP episode received a mean of 16.7 TPE sessions; 59% used rituximab. Among patients with ≥ 1

aTTP episode, exacerbations occurred in 17% (52/302); relapse occurred in 11% (34/302). Mortality rate was 25% (167/666) among all patients with aTTP diagnosis and 14% (41/302) among patients with ≥ 1 aTTP episode. **Conclusion:** Despite treatment with TPE and immunosuppressants, the high mortality and morbidity observed in this patient population demonstrates the need for more effective therapies to improve clinical outcomes.

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STEM CELL TRANSPLANT

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THE ROLE OF SUBPOPULATIONS OF MOBILIZED PERIPHERAL HEMATOPOIETIC STEM CELLS IN THE RESTORATION OF HEMATOPOIESIS DURING HIGH-DOSE CHEMOTHERAPY IN CANCER PATIENTS

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Objective: Mobilized peripheral hematopoietic stem cells are transplanted to cancer patients as support for high-dose chemotherapy. It is believed that the effectiveness of restoring all hematopoietic sprouts during HSC transplantation depends on the total dose of CD34+ cells. At the same time, CD34+ stem cells are a heterogeneous cell pool, including progenitor cells of different levels of differentiation and different ability to proliferate. Accordingly, it can be expected that the subpopulation composition. **Methodology:** We have studied of HSC subsets in 569 specimens of hemopoietic tissue (blood cells and LP cells) from 167 adult cancer patients and on 557 specimens of hemopoietic tissue from 263 pediatric cancer patients. Also, 61 samples of LP from 50 healthy HSC donors were studied. All patients were managed at bone marrow transplantation units of hematology malignancy and oncology department of N.N. Blokhin Cancer Research Center from 1996 to 2014. **Results:** Peripheral hemopoietic stem cells (HSC) that are transplanted to cancer patients to reduce critical pancytopenia vary in subset composition and include early multipotent precursors (CD38- and/or HLA-DR-, CD90+, CD45-negative), lymphoid precursors (CD10+, CD7+, CD2+, CD19+, CD56+), megakaryocyte- (CD61+) and myeloid-committed precursors (CD117+, CD13+, CD33+). These subsets of early and committed HSC are found in different proportions in cancer patients and normal donors. **Conclusion:** So, the pool of mobilized HSC is heterogeneous and represented by pluripotent precursors and committed HSC in different proportions that are in variable, rather sophisticated interrelations. Mobilization effect of SC individual subsets is related with disease type. To achieve fast recovery of granulocyte lineages after HSC autologous or allogeneic transplantation one should not