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A Retrospective Chart Review Study to Evaluate the Clinical Outcome according to Treatment in Atypical Hemolytic Uremic Syndrome Patients in South Korea: A interim analysis

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Objectives: Atypical hemolytic uremic syndrome (aHUS) is a rare disease caused by thrombotic microangiopathy (TMA) due to uncontrolled activation of the complement immune system. Eculizumab that was approved in 2016 is a humanized monoclonal antibody that blocks terminal complement C5 activation. We aim to identify the clinical characteristics of patients in Korea who were diagnosed as aHUS, and the clinical prognosis in patients treated with or without eculizumab.

Methods: In this retrospective, non-interventional, multi-center, chart review study, 57 patients who were clinically diagnosed with aHUS from 1 January 2013 to 31 December 2021 were enrolled at 15 sites in Korea. Of these, 52 patients were included in the analysis set; 12 (23%) in the eculizumab treated group, 40 (77%) in the non-eculizumab treated group. As the primary endpoint we compared the end-stage kidney disease (ESKD)-free survival rate and overall survival rate as markers to evaluate long-term survival rate according to eculizumab treatment, by using the cox-proportional hazard model.

Results: The age of diagnosis of aHUS in patients at baseline was 47.04 years. 55.77% of patients were diagnosed with TMA triggering events and 17.31% had multiple TMA. There was no significant difference between two group except for extra-renal manifestations in various baseline characteristics. The ESKD-free survival probability (78.75% vs 52.03%) and overall survival probability rate (100.00% vs 69.82%) were relatively high in the eculizumab treated group compared to the non-treated group, respectively. However, this effect was not statistically proven due to the small number of enrolled patients.

Conclusions: The results of this interim report support the characteristics of Korean aHUS patients. Furthermore, although not statistically significant, it suggests that eculizumab has the potential to improve survival in patients with aHUS. More data of patients will be collected and evaluated in future planned reports, and the effectiveness of eculizumab will be analyzed from various clinical perspectives.