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SCIENTIFIC LETTER

Optimizing the Long-Term Therapy with Eculizumab (ECU) for Atypical Hemolytic Uremic Syndrome (aHUS)

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To the Editor: Eculizumab has dramatically improved both the patient and kidney survival of subjects with atypical Hemolytic Uremic Syndrome (aHUS) [1, 2]. However, its optimal long-term treatment schedule and duration is still a matter of debate [3]. Here we analyze the long-term clinical course of 6 pediatric patients treated with modified Eculizumab schedule.

Among patients with aHUS followed at the Pediatric Nephrology Unit of the Pediatric Hospital "Giovanni XXIII" of Bari between 01st January 2011 and 30th June 2022, six pediatric patients (5M,1F) with aHUS due to complement-related gene abnormalities and treated with long-term Eculizumab therapy were enrolled (Supplementary Table S1). The main clinical data and blood parameters were collected monthly. All the aHUS patients were treated initially with Eculizumab according to the scheme. After a starting period of 18 mo to reach a stable clinical remission of aHUS, within the next 6 mo the Eculizumab infusion interval was progressively extended up to 32 d (23-40). From 24 mo and during the entire follow-up of 61 mo (5-108) after the extension of the Eculizumab infusion interval, the main hematological and kidney parameters, which were monitored monthly, remained stable (ANOVA p=ns for Platelets, LDH, Hemoglobin and eGFR). Moreover, in the same period no aHUS recurrence was observed, as well as there was no development of end-stage renal disease (ESRD) or the onset of severe adverse events related to the Eculizumab administration.

Although limited to 6 patients, our data confirms the safety and efficacy of long-term Eculizumab therapy for aHUS and supports the possibility to prolong its administration time interval, when associated with careful monitoring of current blood chemistry parameters [4]. This approach might be an effective and safe strategy to optimize the use of Eculizumab and to significantly reduce the cost of therapy and its impact on patients' quality of life.

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Declarations

Conflict of Interest None.

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