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Risk for TMA recurrence and renal outcomes after eculizumab discontinuation in aHUS: results from the Global aHUS Registry

Dr Gema Ariceta[1], Dr Fadi Fakhouri[2], Dr Lisa Sartz[3], Dr Benjamin Miller[4], Dr Vasilis Nikolaou[5], Dr David Cohen[6], Dr Andrew Siedlecki[7], Dr Gianluigi Ardissino[8], Dr Sian Griffin[9]

[1]Vall d' Hebron Hospital, and the Autonomous University of Barcelona, Barcelona, Spain, [2]CHU de Nantes, Nantes, France, [3]Department of Pediatrics, Skane University Hospital, Lund University, Lund, Sweden, [4]Alexion Pharmaceuticals, Inc, Boston, USA, [5]Parexel International, Uxbridge, UK, [6]Columbia University Medical Center, New York, USA, [7]Brigham and Women's Hospital, Boston, USA, [8]Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy, [9]University Hospital of Wales, Cardiff, UK

Background: Eculizumab (Ecu) modifies the course of disease in patients (pts) with atypical hemolytic uraemic syndrome (aHUS), but there are limited data to describe thrombotic microangiopathy (TMA) recurrence rates and long-term outcomes after Ecu discontinuation (d/c).

Methods: Pts in the Global aHUS Registry (NCT01522183) who received ≥1 month (mo) of Ecu with evidence of hematologic or renal response prior to d/c and with ≥6 mo of follow-up (f/u) were included. Those on chronic dialysis (≥3 mo) at the time of Ecu d/c were excluded. Classification as pediatric (<18 years) or adult was made at time of Ecu d/c.

Results: 151 pts (62% female) were included in the analysis: 34% were pediatric and 66% were adults (median [range] age at enrolment, 6.0 [0.6–17.1] and 35.7 [18.4–81.2], respectively), 11% had a family history of aHUS and 41% had a pathogenic variant or anti-CFH antibody. Median (range) duration of Ecu prior to d/c was 1.0 (0.1–5.1) and f/u was 2.3 (0.1–7.1) years. 24% experienced TMA recurrence after Ecu d/c. More pts required antihypertensives at f/u vs at d/c (71% vs 54%). Pts with a family history of aHUS, pathogenic variants, lower eGFR and extrarenal manifestations appeared to be at a higher risk of TMA recurrence (Table).

Conclusions: Discontinuation of Ecu is not without risk and may lead to TMA recurrence in some patients with aHUS. A careful assessment of risk factors prior to the decision to d/c Ecu is warranted.