P330

P330 -Eculizumab Safety: 5-Year Experience From the Global aHUS Registry

Dr Rodney Gilbert[1]
[1]*Southampton Childrens' Hospital, Southampton, United Kingdom*

Background: Eculizumab (ECU) is approved for treatment of atypical hemolytic uremic syndrome (aHUS), a rare and life-threatening disease. The Global aHUS Registry was initiated in 2012 and has recruited both ECU- and non-ECU-treated adult and pediatric patients (pts).

Methods: This observational, multinational Registry (NCT01522183) evaluates safety and effectiveness of ECU in pts with aHUS, while also assessing clinical characteristics at enrollment and follow-up visits every 6 months thereafter, irrespective of disease management. Herein, we report baseline characteristics and targeted safety events from adult and pediatric pts who were “ever treated” (ET) vs “never treated” (NT) with ECU in the first 5 years of the Registry through the study cut-off date of January 26, 2017.

Results: Overall, N=1321 pts (ET, n=865; NT, n=456) have been enrolled. In ET patients, mean (SD) ECU exposure was 2.17 (1.56) years in adults and 2.72 (1.74) years in children. At baseline, ET pts had greater rates of organ involvement (renal: 79.2% vs 23.7%; gastrointestinal: 35.0% vs 9.9%; cardiovascular: 28.3% vs 8.6%; CNS: 25.9% vs 7.9%; and pulmonary: 15.0% vs 6.4%), and poorer quality of life (lower median Functional Assessment of Chronic Illness Therapy–Fatigue scores: 30 vs 41), compared with NT pts, respectively. Prespecified targeted safety events reported for 801 adult and 464 pediatric pts are presented in the Table. No differences in event rates between ET and NT pts were observed, except for serious infections in pediatric pts, which were more common in ET vs NT.

Conclusions: Five-year data from the Global aHUS Registry reveal no new safety signals with ECU treatment in either adult or pediatric ET pts, confirming ECU’s safety profile and, therefore, its positive benefit:risk ratio in a real-world setting.