BLEEDS DURING THE FIRST YEAR OF EMICIZUMAB PROPHYLAXIS: PRELIMINARY RESULTS OF THE BRAZILIAN EMICIZUMAB REGISTRY (EMCASE PROJECT)

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Emicizumab prophylaxis is recommended to prevent bleeds in people with hemophilia A without (PwHA) and with inhibitors (PwHAi). The EMCase Study is a registry of PwHA/PwHAi on emicizumab prophylaxis in Brazil.

We aimed to evaluate the annualized bleeding rates (ABR) for treated bleeds before and during the first year of emicizumab prophylaxis.

Clinical data from previous treatment (last 12 months) and during (first 12 months) emicizumab prophylaxis were collected. Bleeding was classified as spontaneous, post-traumatic, and unknown cause, and only counted if it received any treatment for hemostasis. ABR-total corresponded to the sum of bleeding treated over time. The ABR was annualized for 1 PwHAi due to a follow-up of less than 12 months before starting emicizumab prophylaxis.

A total of 9 PwHA and 43 PwHAi had at least 1 year of emicizumab prophylaxis. Of this, 39/51 (77 %) PwHA/PwHAi were on prophylaxis and all participants in exclusive episodic treatment were PwHAi (12/51; 23%). The median age at the start of emicizumab prophylaxis was 12.6 years. Only 1 (2%) PwHA/PwHAi did not receive an emicizumab loading dose. The most prescribed maintenance regimen was 1.5 mg/kg/week (27/52; 52%). ABR-total reduced from 3.0 (interquartile range/IQR 1.0-5.8; n=52) before emicizumab prophylaxis to 0.0 (IQR 0.0-0.8) in the first year of emicizumab prophylaxis ($p< 0.001$). Similarly, ABR for spontaneous bleeds reduced from 0.0 (IQR 0.0-2.0; n=51) to 0.0 (0.0-0.0; $p< 0.001$), and ABR for post-traumatic bleeds reduced from 1.0 (0.0-3.0; n=51) to 0.0 (0.0-0.0; $p< 0.001$). The number of PwHA/PwHAi with zero bleed increased from 10/52 (19%) to 39/52 (75%) in the previous year and during emicizumab prophylaxis, respectively ($p=0.050$). No adverse events were reported.

Compared to the treatment in the previous year, emicizumab prophylaxis was related to a lower ABR and a higher prevalence of people with zero bleed, without adverse events reports.