Emicizumab in haemophilia A

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Dr Radha Ramanan reports on one of her Australian Haemophilia Centre Directors' Organisation (AHCDO) research projects.

Funded access to emicizumab prophylaxis for certain patients with haemophilia A was approved by the National Blood Authority in November 2020 through the governance of haemophilia treatment centres (HTCs). Emicizumab (Hemlibra®) is a non-factor therapy for haemophilia A that is injected sub-cutaneously (under the skin). This project aims to assess the real-world outcome data since implementation and wide-spread use of funded emicizumab, collected through the Australian Bleeding Disorders Registry (ABDR).

BLEEDING RATE OUTCOMES

We have already collected the data on bleeding rates in patients with haemophilia A and are in the process of publishing this data as a scientific paper. The data was also recently presented in June 2023 at the International Society on Thrombosis and Haemostasis (ISTH) Congress in Montreal, Canada.[1]

In the overall population, we found that patients with haemophilia A have a reduced risk of bleeding since changing to emicizumab. The proportion of patients with zero bleeds increased from 54% to 64% after commencement of emicizumab. However, bleeding risk remained unchanged in adults and those on regular prophylaxis prior to emicizumab.

SURGERY OUTCOMES

Secondly, we aim to assess outcomes in the peri-operative setting in patients with haemophilia A who have switched to emicizumab therapy. The HAVEN-1 and 2 trials included patients undergoing emergency surgery, however excluded those undergoing planned/elective surgical procedures. We have begun collecting this data and have data on 11 patients who have undergone 13 procedures so far. We will continue collecting data until the end of 2024. We will evaluate the laboratory measures of emicizumab if performed, rates of bleeding peri-operatively, adverse effects such as venous thromboembolic events and use of recombinant factor VIII or bypassing therapy in this setting. Preliminary data has been presented at the Australian Blood conference in 2022.

QUALITY OF LIFE OUTCOMES

Thirdly, we aim to collect data on quality of life in these patients using a validated questionnaire called the PROBE questionnaire. This will be sent out via email to participants.

REFERENCES

1. Ramanan R, Parikh S, MacFadyen JD, Tran H. Comparison of intraindividual bleeding outcomes pre- and post-emicizumab in Australian adults and children with haemophilia A with and without inhibitors: a nationwide real-world experience. Abstract PB0194. ISTH 2023 Congress, 24-28 June 2023, Montreal Canada. isth2023.eventscribe.net, accessed 7 August 2023.

Dr Radha Ramanan is the current Australian Haemophilia Centre Directors' Organisation (AHCDO) Research Fellow.