



The 65th ASH Annual Meeting Abstracts

ONLINE PUBLICATION ONLY**322.DISORDERS OF COAGULATION OR FIBRINOLYSIS: CLINICAL AND EPIDEMIOLOGICAL****Prospective Cohort Study on Paediatric and Adult Haemophilia a Patients Correlating Emicizumab Levels with Clinical Experience**

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Aim:

The efficacy of Emicizumab in preventing bleeds in patients with Haemophilia A has been demonstrated in the HAVEN studies. Emicizumab is not universally available at the recommended dosages and there is a push towards reducing frequency and dose to increase availability. This study aims to provide data on Emicizumab levels and corresponding clinical response in the real world.

Method:

Moderate-severe Haemophilia A patients who transitioned to Emicizumab were identified from 2 Centres in Western Australia between November,2017 to December,2022. Bleeds requiring factor replacement were recorded 12 months prior to transition and 12 months following. These were obtained from the Australian Bleeding Disorders Registry and patient hospital notes. Available steady state Emicizumab levels were also recorded.

Results:

31/32 paediatric patients had the same or improved bleeding rates since transition to Emicizumab. No patient had a spontaneous bleed since transition. One patient continued to have high bleeding rates due to adherence issues, with an Emicizumab level of 10µg/ml. Of the 16/32 patients had Emicizumab levels measured with a mean 55.54µg/ml (range 38.8 - 81).

21/24 adult patients experienced the same or improved bleeding rates post transition. 7 patients did not have completed bleed data. 3 patients had worse bleeding rates since transition with 1 bleed each. Their Emicizumab levels ranged 58.54-79.15µg/ml. Emicizumab levels were recorded in 16, with a mean of 53.08µg/ml (range 18.47 - 85.22).

6 adult patients had surgical procedures with 2 bleeds; 1 bleed occurred 10 days after colonoscopy with emicizumab level 18.47ug/mL and 2nd bleed occurred 1 day after pharyngeal level 42.42ug/mL.

Conclusion:

In both paediatric and adult patients, Emicizumab was an effective treatment in preventing bleeds. The mean and range of Emicizumab levels in both groups who had an effective response were similar. The wide range of therapeutic levels may be of important for dosing changes, particularly with the paediatric cohort

Disclosures No relevant conflicts of interest to declare.

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