AHCDO research: VWD and acquired haemophilia A

Caitlin Rice

Dr Caitlin Rice is a fifth-year trainee from Northern Ireland. Caitlin has been working in Australia on an Out of Program Fellowship with the aim of developing clinical experience outside the UK NHS system and gaining further research experience in non-malignant hematology and bleeding disorders. In 2023, Caitlin was located at Royal Perth Hospital with Prof Wendy Erber with a focus on laboratory hematology. In 2024, she transferred to Fiona Stanley Hospital with Dr Stephanie P'ng, commenced in the role of AHCDO Research Fellow and started planning research projects.

Two of Caitlin's research projects are outlined below. Caitlin will work closely with the AHCDO ABDR Research Fellow to utilize data from the ABDR for these projects.

Type 3 von Willebrand disease in Australia: review of outcomes

Type 3 von Willebrand disease (VWD) is a rare bleeding disorder caused by a marked deficiency or absence of von Willebrand factor (VWF). It accounts for < 5% of all patients with VWD and occurs with an approximate incidence of 1 in every 500 000 individuals. According to the most recent report of the Australian Bleeding Disorders Registry (ABDR), there are 148 patients registered with the ABDR who are classified as having severe (type 3) von Willebrand disease.

The management of type 3 VWD is complex and variable. Management guidelines are usually embedded within guidelines of other forms of VWD including the much more common type 1 VWD and there are no guidelines specifically addressing this complex disease.

Primary Outcome

To characterise the clinical and laboratory features of patients in Australia with type 3 VWD and document the current clinical care focussing on the use of prophylaxis and the burden of bleeding events.

Secondary Outcomes

To identify unmet needs in patients with type 3 VWD including treatment options and long-term disease burden.

The Australian experience of haemostatic management in acquired haemophilia A

Acquired haemophilia A (AHA) is a rare and very serious acquired bleeding disorder. The aetiology is of autoantibodies against factor VIII (FVIII/8), which impair FVIII function.

Standard of care treatment utilizes immunosuppressive therapies to suppress autoantibody formation. Another mainstay of treatment is the prevention of injury with risk of subsequent bleeding, and use of bypassing agents to control active bleeding. Haemostatic therapies include recombinant activated factor VII (rFVIIa), activated prothrombin complex concentrate (APCC) or recombinant porcine FVIII (rpFVIII). As haemostatic therapy is expensive and complex, its use should be guided by experienced specialists.

These agents are used irrespective of inhibitor titre and residual FVIII activity - meaning that many patients with life or limb-threatening bleeding will require use of bypassing agents, and often over an extended period of days to weeks. A recent article in *Blood* journal (2021) addressed the use of emicizumab as an alternative haemostatic agent in the management of AHA. The use of emicizumab may have significant cost saving implications due to good haemostatic efficacy, allowing early discharge, and avoidance of expensive bypassing agents for haemostatic control.

Primary Outcome

Record the extent of product use for haemostatic management of AHA in Australia over 5 years (2018-2023).

Secondary outcomes

Quantify the clinical and economic burden of bleeding complications related to choice of haemostatic agents in acquired haemophilia A management.

This may inform cost effectiveness for a business case for consideration of use of emicizumab as first-line therapy for haemostatic management in patients in Australia with AHA.

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Dr Caitlin Rice is the 2024 AHCDO Research Fellow