REAL-WORLD EXPERIENCE WITH EMICIZUMAB: CLINICAL RESULTS AND AN EXCEPTIONAL CASE OF TYPE III VON WILLEBRAND DISEASE WITH INHIBITOR

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Background and importance: Emicizumab is a humanized bispecific monoclonal antibody that mimics the cofactor function of activated factor VIII and is primarily indicated for the prophylactic treatment of patients with hemophilia A, regardless of the presence of inhibitors. Although its use in other inherited bleeding disorders, such as von Willebrand disease, remains limited and off-label, it is increasingly being explored as a therapeutic alternative. We present our practical experience in a tertiary care center, highlighting a notable case of type III von Willebrand disease with an inhibitor, in which the use of emicizumab produced promising clinical results.

Aim and objetive: Evaluate the efficacy, safety, and clinical applicability of emicizumab in patients with hereditary coagulopathies, with special attention to a case of type III von Willebrand disease with an inhibitor.

Material and methods: This is a retrospective, single-center observational study involving eight patients treated with emicizumab between December 2019 and May 2025. Clinical, genetic, and follow-up variables were collected, including diagnosis, age at treatment initiation, presence of inhibitors, history of bleeding episodes, bleeding events after treatment, surgical or invasive procedures, and adverse reactions.

Results: The cohort included 7 patients with severe hemophilia A (one with an inhibitor) and one patient with type III von Willebrand disease with an inhibitor. The mean age at the start of treatment was 22 years (range: 5–40). All patients had a history of clinically significant bleeding episodes. After starting emicizumab, six patients experienced no further bleeding episodes. Mild episodes (epistaxis and muscle hematoma) were reported in 2 cases, neither of which required additional treatment.
The 19-year-old patient with von Willebrand disease, who had a history of epistaxis and deep hematomas, showed a favorable response, with only one mild episode of epistaxis during follow-up. No serious adverse effects were reported, and only one patient experienced mild transient headache.

Conclusion and relevance: Emicizumab proved to be effective and safe in this cohort of patients with hemophilia A, including those with inhibitors. It is noteworthy that the case of the patient with type III von Willebrand disease and an inhibitor demonstrated excellent tolerability and bleeding control. These findings support the potential role of emicizumab as a prophylactic treatment in bleeding disorders beyond hemophilia A.

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