6. EMICIZUMAB IN PEDIATRIC HEMOPHILIA A: A CASE STUDY

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Introduction. The development of inhibitors that neutralize the function of clotting factor VIII is currently the most challenging complication associated with the hemophilia A treatment. Emicizumab is a humanized bispecific monoclonal antibody that is designed to substitute the hemostatic function of activated FVIII by bridging activated factor IX and factor X to activate FX, thereby facilitating the coagulation cascade and achieving hemostasis in patients with hemophilia A. Due to this mechanism of action, severe HA is changed into a mild form with an estimated FVIII activity of at least 9%.

Case statement. This study explores emicizumab intervention in a one-year-old with hemophilia A and inhibitors. Clinical records, labs, and imaging assessed bleeding severity. Emicizumab, following evaluation of history and titers, showed a reduction in bleeding frequency and severity. Improved clinical outcomes, joint health, and quality of life were observed. The study, addressing gaps in hemophilia A management in infants, emphasizes early diagnosis and personalized treatment.

Discussions. Emicizumab was licensed for bleeding prophylaxis in PWHA with inhibitors and is thus the first approved non replacement therapy. Emicizumab prophylaxis resulted in a markedly reduced annual bleeding rate. After a loading phase of 4 weeks with a dose of 3 mg/kg body weight (BW) weekly , the maintenance therapy can be performed with 1.5 mg/kg BW weekly , 3 mg/kg every 2 weeks or 6 mg/kg BW every 4 weeks.Emicizumab is associated with beneficial effects on health-related quality of life and health status, and is generally well tolerated

Conclusion. Emicizumab's promising results highlight its potential in revolutionizing hemophilia A with inhibitors in infants, contributing to growing evidence supporting its efficacy in this challenging population