

EVALUATION OF SAFETY AND EFFICACY OF EMICIZUMAB PROPHYLAXIS IN PEDIATRIC PATIENTS WITH HEMOPHILIA A - A SINGLE CENTRE EXPERIENCE.

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Background and Aims: Hemophilia A (HA) is an hereditary X-linked bleeding disorder secondary to deficiency of the clotting factor VIII (FVIII), that, if not properly treated, is associated with debilitating joint damage due to recurrent hemarthroses as well as life-threatening bleeds including intracranial hemorrhage. The introduction of prophylaxis therapy with emicizumab, a monoclonal antibody that replaces the function of the activated FVIII and prevents bleeding, has changed the burden and improved the quality of life of haemophilic patients, mainly in the pediatric population. However, there is a scarcity of data about its use in patients with HA. We aimed to evaluate the safety and efficacy of emicizumab prophylaxis in pediatric patients with severe HA.

Methods: Patients with severe HA were enrolled at our hospital. They take emicizumab weekly (3 mg/kg) for a month and then went into a maintenance period with a different dosing regimen. Breakthrough bleeding episodes and the annualized bleeding rate (ABR) were reported for all patients before and after emicizumab prophylaxis. Also, all adverse events during prophylaxis were documented to evaluate the safety of emicizumab.

Result: In total, 28 pediatric patients with severe HA were

enrolled in this study, from 2018-2019. Emicizumab prophylaxis was started in 2018-2019 in patients with inhibitor and, subsequently, from 2020 also in patients without inhibitor. The median patients' age was 7, 5(1-15) years. Before starting therapy with emicizumab median annualized bleeding rate was 1.195. After switching to emicizumab, median annualized bleeding was 0. Six patients received their first dose within the first hours of life, among these five never had a bleeding episode. No serious side effects were reported among patients, only reactions at the infusion site.

Conclusion: Prophylaxis using emicizumab was associated with a significantly lower bleeding rate in HA patients. The majority of patients had zero bleeds with emicizumab prophylaxis. Therefore, Emicizumab is efficacious and well tolerated in infants, due to a less invasive administration route which increases compliance by the patient and parents and solves the problem of difficult venous accessibility in pediatric patients. Furthermore, emicizumab improve quality of life in children and caregivers, by reducing pain and bleeding and, as a consequence, needing for factor replacement and lower treatment burden for family. Moreover, this type of prophylaxis is associated with better emotional well-being, less anxiety over bleeds, fewer missed school days, changing the daily life for these patients and their families.

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