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An Institutional Experience With Emicizumab In Pediatric Patients With Heophilia A

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AN INSTITUTIONAL EXPERIENCE WITH EMICIZUMAB IN PEDIATRIC PATIENTS WITH HEMOPHILIA A

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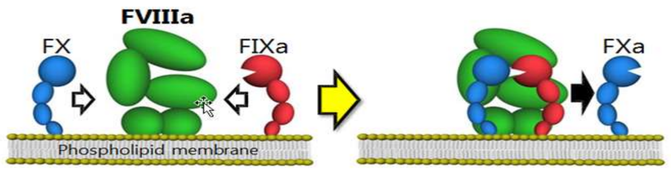
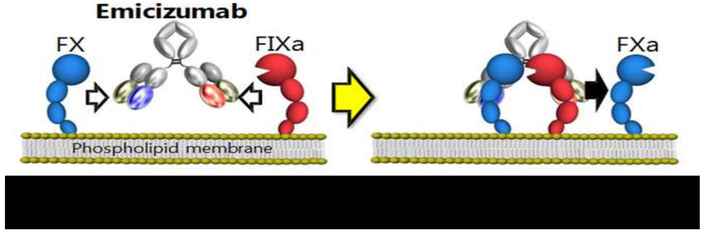
Background

•Emicizumab is a recombinant, humanized, bispecific monoclonal antibody that substitutes for the function of FVIII by binding to activated factor IX and X. It is currently indicated for routine prophylaxis in adults and children of all ages with hemophilia A, with or without inhibitors. Despite basic guidelines provided by MASAC and the drug package insert, there is significant inter-institutional variability regarding monitoring and follow up in patients on emicizumab.





Objectives

•To outline our institutional experience in pediatric patients on **Emicizumab**

Methods

•Retrospective and prospective chart review of our patients on emicizumab. Information collected included age of patient, hemophilia severity, presence and titer of inhibitors, age when emicizumab was initiated and bleed management.

Results

•24 pediatric patients, All male patients
•Ages: 11mo-16y
•Severity: 4 with moderate(M), 19 severe(S), 1 mild (m)
• 3 patients with inhibitors (2 with high titer)

age at starting emicizumab (year)	Hemophilia severity	Presence of inhibitor (Y/N)
7	m	Y
2	S	N
10	M	N
16	M	N
11mo	S	N
12	S	N
12	S	N
11	S	N
12	S	N
5	S	N
2	S	N
7	S	N
7	S	N
6	S	N
10	S	N
2	M	N
13	S	N
12	S	Y
8	S	Y
6	S	N
15	S	N
1	S	N
7	M	N

• Labs included chromogenic FVIII activity and inhibitor within a month prior to starting therapy. Repeat levels are done at 3mo and then annually. Initial dose with teaching is done in clinic. Parents have the option to follow up weekly if needed.

•Scheduled visit are at week 5, 3mo, 6mo and 6 monthly thereafter. PTT is checked at each follow up. An educational checklist is completed by the provider and a nurse at the initial visit. Dose is recalculated at each visit based on weight.

•FEIBA is noted as a drug allergy in the patient's chart. Central lines are removed after 3mo of emicizumab therapy.

•Single dose of factor product at 50 IU/kg or recombinant factor VII at 60-70mcg/kg has provided adequate hemostasis for minor surgical procedures. Most dental procedures have required only anti-fibrinolytic therapy.

•Majority of joint and soft tissue injuries were managed with RICE alone or a single dose of factor if there is a bleed. Most patients are on q2 or q4 week dosing for maintenance based on convenience and number of injections needed at a time.

Discussion

•Emicizumab can be administered subcutaneously and less frequently than factor VIII products

•>95% reduction in annual bleed rates reported

• Patients report significantly improved joint health and quality of life

•The custom educational check list at our HTC has proven to be the most useful tool for our staff, patients and families.

•This has ensured adequate teaching and uniform monitoring for all the patients. A multi-institutional effort would help establish emicizumab monitoring guidelines for patients across the globe.

References

•Treatment of bleeding episodes in haemophilia A complicated by a factor VIII inhibitor in patients receiving Emicizumab. Interim guidance from UKHCDO Inhibitor Working Party and executive committee

P.W.Collins et al, *Haemophilia March 2018*

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