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ABSTRACT N. PO24

EMICIZUMAB PROPHYLAXIS AND BLEEDING OUTCOMES: A SINGLE CENTER CLINICAL EXPERIENCE.

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Background and Aims: Emicizumab for hemophilia A (HA) has been increasingly employed in clinical practice. The aim is describing our real-world experience and clinical outcomes in severe HA patients (pts).

Methods: Retrospective, single-center study including severe HA pts, with/without inhibitors, with 6-month minimum follow-up (FU) since emicizumab start. Clinical data were collected from clinical charts and all participants signed an informed consent form.

Results: We enrolled 29 severe HA pts who switched to emicizumab prophylaxis (EP). The only adverse event we recorded was a local skin reaction in 1 pt after his 1st emicizumab dose.

Non-Inhibitor patients: 24 pts (82.8%), whose median age at switch was 14.1 years (7.43-38.33; min 0.65-max 55.33) and median FU is 16.97 months (9.6-27.2). Before EP, 3 (12.5%) were therapy naive, 2 (8.3%) treated on-demand, 19 (79.2%) were on recombinant factor VIII (rFVIII) prophylaxis. The main reasons for switching were achieving better bleeding control, avoiding intravenous infusion, and improving compliance. During EP, 1 pediatric pt, who had previously received few rFVIII infusions, developed an inhibitor following a traumatic injury that required rFVIII treatment.

Patients with inhibitors (PwI): 5 pts (17.2%), whose median age at switch was 58.1 years (37.95-67.7) and median FU is 51.7 months (37.63- 73.75). All were treated with on-demand recombinant activated factor VII (rFVIIa).

Procedures: during EP, 11 procedures were performed in 7 (13.8%) pts (4 PwI, 3 non-inhibitor): 4 major surgeries, 3 minor ones, 4 dental extractions. Major surgeries were managed with rFVIII/FVIIa, yet 1 PwI presented postoperative bleeding complications (melena after pancreatic biopsy) that required transfusions and large amounts of rFVIIa.

Bleedings: During the 2 years prior to switch, 25 (85.2%) pts experienced at least one bleeding episode (5 PwI and 20 non-inhibitor). Comparing the mean annualized bleeding rate (ABR) and annualized joint bleeding rate (AJBR), PwI had significantly higher rates (p=0.0116 and p=0.0092). During EP, 14/29 (48.3%) pts never experienced a bleeding episode. 11 non-inhibitor pts experienced 19 mild-moderate bleeds: 17 traumatic and 2 spontaneous hematuria (same pt), all managed with rFVIII therapy. 4 PwI experienced a total of 5 mild-moderate bleeds: 4 traumatic (managed with rFVIIa) and 1 small spontaneous hematoma. Comparing pre-vs-post emicizumab ABR and AJBR, we observed a reduction by 63.78% (p=0.0114) and 56.52% (p=0.17) in the non-inhibitor group and a reduction by 96.17% (p=0.0079) and 98.57% (p=0.0476) in PwI. No significant difference was found in ABR and AJBR between PwI and non-inhibitor during EP (p=0.69 and p=0.63, respectively). During EP, ABR and AJBR did not differ between pediatric (age<12 years, 11 pts) and adult pts (age>12 years, 18 pts).

Conclusions: Despite our small cohort, we confirmed emicizumab efficacy and safety in severe HA pts with/without inhibitor. The different age and FU between the pt groups depend on timelines of regulatory indications for prescription. Only 2 pts had mild spontaneous bleeds and traumas were all easily managed. 1 PwI had severe bleeding after a high-risk procedure, despite additional rFVIIa. We found no significant differences in bleeding rates between pt groups during EP. We believe that the smaller AJBR reduction in the non-inhibitor group may be explained by a more confident approach to physical activity by pediatric pts.

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Clinical characteristics and outcomes			
Variable	**HA without inhibitors**	**HA with inhibitors**	**p-value**
No. patients	24 (100%)	5 (100%)	-
Median age, *years* (IQR)	14.1 (7.43 - 38.33)	58.1 (37.95 - 67.70)	**p=0.0066**
Median FU, *months* (IQR)	16.97 (9.60 - 27.20)	51.70 (37.63 - 73.75)	**p<0,0001**
Previous treatment			-
none	3 (12.5%)	-	
rFVIII			
On-demand	2 (8.3%)	-	
Prophylaxis	19 (89.2%)	-	
rFVIIa (on-demand)	-	5 (100%)	
Inhibitor history	5/24 patients (20.8%)	-	-
low-titer	2 (8.3%)		
high-titer	3 (12.5%)		
Current inhibitor	-		-
median pre-emi titer (IQR)		42.0 (10.1 - 1997.0)	
low-titer (< 5 BU)		0 (0%)	
high-titer (≥ 5 BU)		5 (100%)	
Previous ITI	4/24 patients (16.7%)	2/5 patients (40%)	-
successful	4 (100%)	0 (0%)	
unsuccessful	0 (0%)	2 (100%)	
Pre-emi bleedings (2 y)	20/22 patients (90.9%)	5/5 patients (110%)	
total events	50	45	
ABR, average	1.27 (CI 0.66; 1.88)	4.70 (CI 0.62; 8.79)	**p=0.0116**
AJBR, average	0.46 (CI 0.14; 0.77)	4.2 (CI -0.87; 9.27)	**p=0.0092**
Bleedings during emi	11/24 patients (45.8%)	4/5 patients (80%)	
total events	19 (100%)	5 (100%)	
spontaneous	2 (10.5%)	1 (20%)	
traumatic	17 (89.5%)	4 (80%)	
ABR, average	0.46 (CI 0.18; 0.74)	0.18 (CI -0.04; 0.40)	p=0.6894
AJBR, average	0.20 (CI 0.02; 0.37)	0.06 (CI -0.10; 0.21)	p=0.6304
Surgical Procedures	4 procedures (100%)	7 procedures (100%)	-
Major	2 (50%)	2 (28.6%)	
Knee arthroplasty	1	1	
Pancreatic biopsy	0	1	
Skin lesion removal	1	0	
Minor	2 (50%)	1 (14.3%)	
Colonoscopy	0	1	
CVC removal	1	0	
Cataract	1	0	
Dental extractions	0 (0%)	4 (57.1%)	
Bleeding complications	0 (0%)	1 (14.3%); post-biopsy melena	