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ABSTRACT N. PO49

USE OF EMICIZUMAB IN TWO PATIENTS WITH ACQUIRED HEMOPHILIA A: A NEW THERAPEUTIC APPROACH.

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Background

Emicizumab is a humanized bispecific monoclonal antibody that mimics activated factor VIII (FVIII) and is currently approved as prophylaxis for patients with congenital severe and moderate hemophilia A, with or without inhibitors. Acquired hemophilia A (AHA) is a very rare bleeding disorder caused by the development of autoantibodies against FVIII, leading to severe and spontaneous bleeding, either cutaneous or intramuscular. AHA is often secondary to conditions such as cancer, autoimmune diseases, or infections. Standard therapy involves immunosuppressive treatment aimed at eradicating the inhibitor, which can be complicated by infections especially in older frail patients, and agents to manage acute bleeding; these include recombinant activated factor VII (rFVIIa), activated prothrombin complex concentrate (aPCC), and recombinant porcine FVIII concentrate (rpFVIII). Recent case reports and case series have shown clinical improvement in AHA patients treated off-label with emicizumab, indicating a promising new therapeutic option. In Italy, its use is permitted under AIFA's law 648/1996.

Case report

The first case (Figure 1A) involves a female patient born in 1952, diagnosed with AHA in 1978. Over the first three decades following diagnosis, she was treated with corticosteroids and cyclophosphamide, without inhibitor eradication, and for acute bleeding episodes she received rFVIIa and porcine FVIII. In 2014, she was referred to our department. At that time, FVIII level was 0.3% with an inhibitor titer of 83 BU. Treatment with rituximab was started, leading to partial disease control (FVIII ~ 5%; inhibitor ≤10 BU), maintaining disease stability for years with only occasional gingival or post-dental bleedings. In March 2024, however, she experienced a severe bleeding after a breast fine-needle biopsy. Despite aPCC treatment, bleeding persisted (FVIII 5.8%; inhibitor 3.55 BU). Due to the inadequate bleeding control, emicizumab was started in May 2024 at 3 mg/kg weekly, then every two weeks. After one year, no further bleeding episodes occurred. During this period she underwent 2 dental extractions managed with only 2 doses of rFVIIa with an optimal outcome.

The second case (Figure 1B) involves a 60-year-old female diagnosed with AHA in 2023 after bleeding from a chronic lower limb ulcer. She started corticosteroid therapy, followed shortly by systemic antibiotic treatment due to ulcer infection. She achieved a FVIII level of 40% with an inhibitor titer of 1.72 BU. During steroid tapering, she had frequent episodes of epistaxis, treated with rFVIIa. In September 2024, after steroid discontinuation, she developed severe anemia (Hb 5.4 g/dL) due to spontaneous intramuscular bleeding, which was treated with aPCC (FVIII 10.5%, inhibitor 8.9 BU). Steroids were reintroduced, and emicizumab was started at 3 mg/kg weekly, with the aim of either controlling bleeding or discontinuing steroid therapy to promote ulcer healing. After 4 doses, it was stopped because FVIII level rose to 152%, with complete inhibitor disappearance. Steroids were discontinued in January 2025, and to date, the patient has not experienced any further bleeding episodes.

Conclusions

These cases suggest that emicizumab is effective in AHA patients, and it can be very useful to manage difficult situations in fragile patients or in those rare cases who have not eradicate the inhibitor.

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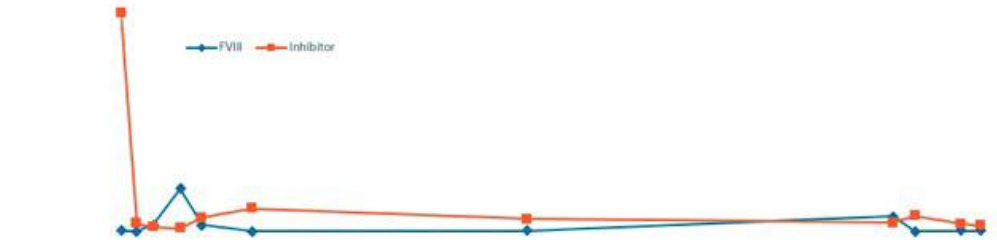
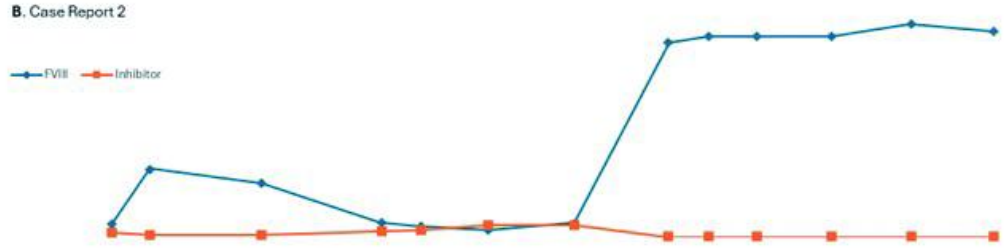
A. Case Report 1



	27/01/2014	11/04/2014	30/06/2014	05/11/2014	16/02/2015	14/10/2015	21/04/2019	07/03/2024	20/06/2024	28/01/2025	29/04/2025
FVIII (%)	0.3	0.25	2.4	16.3	2.5	0.25	0.4	5.8	0.2	0.3	0.4
Inib (BU)	83	3.3	1.84	1.24	5.3	8.9	4.8	3.55	5.95	2.9	2.18
Hb (gr/dL)	12	12.1	12.5	12.2	11.6	12.4	11.8	12.4	12.5	12.7	13.1

B. Case Report 2



	11/12/23	02/01/24	07/03/24	15/05/24	07/06/24	16/07/24	04/09/24	29/10/24	21/11/24	19/12/24	31/01/25	18/03/25	05/05/25
FVIII (%)	10.2	52	41	10.9	7.7	5.5	10.5	148	152	152	152	161.8	156
Inib (BU)	3.38	1.56	1.72	4.3	5.45	9	8.9	0	0	0	0	0	0
Hb (gr/dL)	12	10.8	11.7	9.6	8.8	9.2	5.4	12.3	9.4	11	9.8	11	11.4

Time course of factor VIII levels, inhibitor titre, and hemoglobin values in the first case report (A) and in the second one (B)