2.01 Hemophilia - clinical and treatment issues

INNOVATIVE USE OF EPTACOG BETA TO TREAT ACUTE ELBOW HEMARTHROSIS IN A PEDIATRIC PATIENT WITH HEMOPHILIA B AND A HISTORY OF INHIBITORS, UNDERGOING SECONDARY PROPHYLAXIS WITH CONCIZUMAB

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Background. People with severe hemophilia B (PWBH) require intravenous anti-hemorrhagic prophylaxis with coagulation factor IX (FIX) to prevent acute bleeding and chronic arthropathy. The development of inhibitory antibodies against FIX occurs in 3–5% of PWBH, compromising patient management, particularly in the pediatric population. Although immune tolerance induction (ITI) is the standard of care for inhibitor eradication, outcomes are variable and the occurrence of adverse reactions can compromise treatment success. In these scenarios, bypassing agents, such as recombinant activated factor VII (rFVIIa), is crucial for managing acute bleeding episodes rather than for prophylaxis. Alongside Eptacog alfa, the historical rFVIIa approved as a bypassing agent, Eptacog beta is a novel rFVIIa, potentially less thrombogenic, approved by EMA for PWHB aged over 12 years. Eptacog beta differs from eptacog alfa in its N-glycosylation profile, which can result in greater efficacy. We describe the first use of Eptacog beta to treat bleeding in a PWHB with INH and anti-TFPI prophylaxis.

Case Presentation. We report the case of an 11-year and 4-month-old male patient affected by severe hemophilia B (FIX <1%), with documented high-titer inhibitors and a prior history of an anaphylactoid reaction to a few exposure doses of rFIX. The patient's clinical history was characterized by almost ten years of on demand treatment with Eptacog alfa (ABR=4, target joint: right elbow). In October 2025, to control bleeding episodes he started secondary prophylaxis with Concizumab (a subcutaneous anti-TFPI antibody); no bleeding events were reported in the following three months. In January 2026, due to a new right elbow hemarthrosis, intravenous Eptacog-beta (Cevenfacta) was administered, off-label due to age, following parental informed consent, at a dosage of 75 μg/kg, according to the following regimen: three doses every 3 hours, followed by two doses every 4 hours and a final dose 6 hours later. Treatment with Eptacog-beta induced a rapid hemostatic response, leading to the full resolution of pain and functional impairment. The infusion was well tolerated by the patient; no adverse or hypersensitivity reactions, thrombotic events or development of anti-drug antibodies were reported during hospitalization or throughout the subsequent 90-day follow-up period.

Conclusions. This report represents the first documented evidence in literature of Eptacog beta use in a pediatric patient aged < 12 years undergoing Concizumab prophylaxis. Despite current EMA restrictions in the pediatric population, the favorable safety profile and good tolerability observed suggest that Eptacog beta may represent a valid therapeutic option for managing acute bleeding episodes in clinically complex patients