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EFFICACY AND SAFETY OF EPTACOG BETA (RECOMBINANT HUMAN FVIIA) ACCORDING TO AGE IN PERSONS WITH HAEMOPHILIA A/B WITH INHIBITORS UNDERGOING SURGICAL PROCEDURES

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PO145 | EFFICACY AND SAFETY OF EPTACOG BETA (RECOMBINANT HUMAN FVIIA) ACCORDING TO AGE IN PERSONS WITH HAEMOPHILIA A/B WITH INHIBITORS UNDERGOING SURGICAL PROCEDURES

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Introduction: Eptacog beta (CEVENFACTA®) is a new rFVIIa approved by the EMA for the treatment of bleeding events and prevention of bleeding during surgery in persons with haemophilia A/B with inhibitors (PwHABI) aged ≥12 years (y).

Methods: PERSEPT 3 was a Phase 3 (NCT02020369) trial of eptacog beta in PwHABI who required surgical procedures. Eptacog beta was administered at an initial dose of 200μg/kg or 75μg/kg for major or minor procedures respectively. This was followed by 75μg/kg for ≥5 (major procedures) or ≥2 (minor procedures) days. Haemostatic during the intra and postoperative care period (primary efficacy endpoint was determined by the investigator at the study centre 48±4h after the last dose of eptacog beta, based on the totality of the assessments performed on the patient (pt) at each timepoint). This post-hoc analysis compared the efficacy and safety of eptacog beta by age (pts aged <12 vs ≥12y).

Results: Twelve pts were included (<12y: n=5, 1 major and 4 minor procedures; ≥12y: n=7, 5 major and 2 minor procedures). The primary endpoint success proportion was 100% (95% CI: 39.8–100) in pts aged <12y (4 successes, 1 missing) and 71.4% (95% CI: 29.0–96.3) in pts aged ≥12y (5 successes; 2 failures). The intraoperative success proportion was 100% (95% CI: 47.8–100) for pts aged <12y (5 successes) and 100% (95% CI: 59.0–100) for pts aged ≥12y (7 successes). The success proportion 24h post-procedure was 100% (95% CI: 47.8–100) for pts aged <12y (5 successes) and 100% (95% CI: 47.8–100) for pts aged ≥12y (5 successes; 2 missing). Two pts discontinued treatment (1 aged <12y withdrew consent; 1 aged ≥12y due to an adverse event (AE): postprocedural hematoma). One pt experienced 2 serious AEs leading to death, both were considered unrelated to the treatment. No allergic or thrombotic events occurred; no neutralising antibodies were detected. Antifibrinolytics were used concomitantly with eptacog beta in 4 patients without any safety concerns.

Discussion/Conclusion: This post-hoc subgroup analysis shows that eptacog beta is effective and well-tolerated in perioperative care irrespective of patient age (<12 vs ≥12y), supporting the use of eptacog beta for bleed management (prevention and treatment) in major and minor surgical procedures in all PwHABI.

Disclosure of Interest: None declared

PO146 | RARE BONE COMPLICATION IN A PATIENT WITH CONGENITAL AFIBRINOGENEMIA

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Introduction: Congenital afibrinogenemia is a rare autosomal recessive and hereditary disease with unusual clinical manifestations ranging from moderate bleeding to catastrophic hemorrhage(1). Coagulation tests and bleeding of the umbilical cord stump after birth may be prolonged, and there may be abnormalities in platelet function in these patients(2). Bleeding in the brain (intracranial hemorrhage is rare) or other internal organs can happen that leads to fatality(4).)Patients with frequent bleeding episodes, CNS hemorrhage, or women who have had recurrent miscarriages during pregnancy might receive prophylactic treatment(10).

Methods: A 10-year-old boy patient was referred with case afibrinogenemia, complaining of bone pain in the legs. Over the past two years, he suffers from bone pain in the legs area. Due to the