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**FACTOR XIII DEFICIENCY: CLINICAL  
MANIFESTATIONS AND TREATMENT**

**Background:** FXIII deficiency is a very rare coagulation disorder. Bleeds are usually muscular or mucocutaneous, but bleeding from umbilical cord at birth and intracranial hemorrhages (ICHs) can occur in cases of severe disease. Plasma-derived concentrates (Fibrogammin®) and rFXIII concentrates (Novothirteen®) can be used on demand to control the bleeding. The best treatment in patients with severe disease is the use of plasma-derived or recombinant FXIII concentrates on prophylaxis at the dose of 10-26 IU/kg every 4-6 weeks and 35 IU/kg every 4 weeks, respectively. Few data on the use of rFXIII in the real-world scenario are available.

**Material and methods:** recently we enrolled all patients presenting FXIII deficiency treated with catridecacog at ten Italian Hemophilia Centers. PK-profiles were evaluated and clinical data and outcomes were collected and analyzed.

**Results:** overall 20 patients with FXIII deficiency were enrolled, 75% presenting severe disorder. 11/20 were females. Mean age at diagnosis was 15 years (range: birth-74 years). 60% had a known family disorder. Pharmacokinetics was assessed in 18/20 of cases before starting prophylaxis. Mean age at PK-evaluation was 36.4 years (6-74 years), mean dose of drug infused for PK was 33.9 IU/kg (25-50 IU/kg). Prophylaxis was subsequently started on 65% of patients at a mean dosage of 33.8 IU/kg (range 25.0-80.0 IU/kg), on average every 4.0 weeks (range 3.0-8.0 weeks). During an average follow-up of 43 months, one ileo-psoas hematoma which quickly resolved, one muscular hematoma, and two minor surgeries were reported. One severe patient who remained on demand treatment experienced a severe intracranial hemorrhage.

**Conclusion:** efficacy and safety of prophylaxis with catridecacog was proven in all patients, also in preventing severe bleeding. The cumulative PK profile was similar to that reported in the MENTOR studies, but dosage and infusion timing for each patient were in some cases very different.

