2.07 Novel therapeutic products and approaches (including gene therapy)

SUCCESSFUL TREATMENT WITH EFANESOCTOCOG ALFA OF INTRACRANIAL HAEMORRHAGE IN A NEWBORN AFFECTED BY SEVERE HEMOPHILIA A

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Background. Intracranial haemorrhage (ICH) in the neonatal period is a non-rare and potentially life-threatening complication of haemophilia A. Use of ultra extended half-life factor VIII concentrate (UHL-FVIII) Efanesoctocog Alfa can be crucial in this setting, but until now there is no published experience in this clinical scenario.

Case Report. A term male neonate was born after a dystocic vaginal delivery with vacuum extraction. Initial postnatal adaptation required CPAP support, followed by clinical improvement. On day 3 of life, because of pallor and subcyanotic appearance suggestive of anaemia, cranial ultrasound raised suspicion of intraventricular bleeding and the patient was transferred to the neonatal intensive care unit. Coagulation testing performed that evening revealed an isolated prolonged aPTT ratio of 3.21 (HemosIL SynthaSil ® Werfen) and severely reduced factor VIII activity of 0.2% (HemosIL SynthaSil ® Werfen), consistent with diagnosis of severe haemophilia A. No family history of haemophilia was reported along the maternal lineage, and the mother had no known bleeding phenotype. Brain CT then demonstrated a substantial haemorrhage in the fourth ventricle, with additional mild blood deposition in the occipital horns and along the tentorium and retrocerebellar region, without intraparenchymal bleeding. Efanesoctocog Alfa was administered on day 4 of life (50 IU/kg). On day 6, approximately 40 hours after the first infusion, factor VIII activity was 74.7% (HemosIL Synthafax ® Werfen), supporting a largely sustained haemostatic coverage. A second infusion at the same dose was administered on day 7 of life. In the meantime, MRI reassessment showed a mild reduction of the intraventricular haemorrhage, with no neurosurgical indication. On day 8, follow-up cranial ultrasound confirmed mild bilateral ventricular dilatation without signs of hypertension and no new bleeding sites. Because of difficult peripheral venous access, a midline peripherally-inserted venous catheter was placed on day 10, when a third infusion of Efanesoctocog Alfa was administered. Emicizumab prophylaxis is planned to be started once the haemorrhage is clearly resolving, with the aim of subsequently discontinuing Efanesoctocog Alfa after the second loading dose.

Conclusions. To our knowledge, this is the first case of Efanesoctocog Alfa use in a severe haemophilia A newborn with ICH. Our case suggests that Efanesoctocog Alfa represents an innovative and effective strategy, offering prolonged haemostatic coverage with a reduced number of infusions in an exceptionally fragile clinical setting.