Assessment and validation of a defined fluid restriction in the use of subcutaneous desmopressin (DDAVP) for children with inherited bleeding disorders

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INTRODUCTION

- Desmopressin (DDAVP) has been used in children with von Willebrand Disease (VWD) and mild haemophilia A (HA) for over 30 years
- DDAVP has lowered the number of children exposed to human plasma derived products with a good safety profile and low cost
- Hypoanaemia is a rare but potentially dangerous side effect of DDAVP that is thought to be more frequent in young children
- Fluid restriction around DDAVP use is known to reduce the risk of hypoanaemia but no specific paediatric guideline exists

STUDY AIMS

- Primary aim: assess whether a 2/3 maintenance fluid restriction was effective in preventing hypoanaemia in children receiving subcutaneous (SC) DDAVP in the course of both formal DDAVP trials and therapeutically in the perioperative period
- Secondary aim: demonstrate adequate biological and clinical responses to SC DDAVP

PATIENTS AND METHODS

- A retrospective chart analysis was undertaken of all children who were prescribed DDAVP in our institution between 2008 and 2013
- The subgroup of children who underwent a formal SC DDAVP trial as per our institutional protocol were identified and biological response as well as side effects documented
  - Biological response in haemophilia A was defined as follows: complete (FVIII C > 80% normal), partial (FVIII C 0.5-0.8% normal) or no response (FVIII C < 0.5%) 90 minutes after DDAVP administration
  - Biological response in VWD was defined as at least a 3-fold increase of baseline levels of FVIII:C and VWF:RCo with post levels of at least 0.35 U/ml 90 minutes after DDAVP administration
- Children who used DDAVP for perioperative bleeding prophylaxis were identified and haemostasis outcomes and side effects assessed. In these children daily weights, adherence to the prescribed fluid restriction and formal electrolytes (where measured) were documented

RCH DDAVP PROTOCOL

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SUMMARY

- A two thirds maintenance regimen prevented significant hypoanaemia in our cohort, and is now the standard protocol for fluid restriction post DDAVP administration in our centre
- A trial dose of SC DDAVP produced a complete response in all but one patient with definite or possible type 1 VWD (97% of patients tested)
- A complete or partial response was achieved in 94% of patients with mild HAD
- Planned perioperative use of SC DDAVP provided adequate haemostasis in all patients (no requirement for vWF or FVIII concentrate)
- SC DDAVP was well tolerated – no serious side effects were observed
- Although not formally assessed, our institutional experience suggests that use of SC DDAVP is generally more acceptable to the child and family (compared to our previous standard intravenous protocol)

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