

Desmopressin in moderate hemophilia A patients: a treatment worth considering

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SUPPLEMENTAL MATERIALS

	All patients	No absolute response	Partial absolute response	Complete absolute response
Treatment, n	9	4	3	2
Reason for treatment, n				
Dental procedure	3	-	2	1
Bleeding	5	3	1	1
Unknown	1	1	1	1
Bleeding location, bleeding severity and treatment outcome*				
Hand, major, poor	1	1	-	-
Ankle, minor, moderate	1	-	1	-
Tongue, major, moderate	1	-	-	1
Unknown, unknown, moderate	1	1	-	-
All unknown	5	2	2	1

Supplemental Table 1. DDAVP treatment outcome and absolute response in moderate patients. This table displays the treatment-related DDAVP administrations in our cohort. *Treatment outcome was defined as follows:

- Poor: severe, uncontrolled bleeding did not stop (or was not prevented in case of surgery) within 24 hours and required additional therapy (other than DDAVP).
- Moderate: moderate bleeding in excess of what would be expected for a patient without a bleeding disorder; could not optimally be treated or prevented.
- Adequate: adequate control/prevention of bleeding, blood loss did not exceed what would be expected in normal hemostasis. Bleeding stopped within a few hours and did not recur after one administration. The patient did not require additional product or unplanned treatment.

Results:

Nine patients received DDAVP for treatment of which eight administrations were subcutaneous and one was intravenous. Three patients received DDAVP for a dental procedure, of whom the therapeutic response to DDAVP was unknown. The absolute response was partial in two patients and complete in one patient. Five patients were treated for a bleeding with DDAVP. One patient had a minor bleeding in his ankle. The therapeutic response to DDAVP was moderate and absolute response to DDAVP was partial in this patient. Two patients had a major bleeding, one in his tongue and the other in his left hand with respectively a moderate and poor therapeutic response. The patient with the moderate therapeutic response showed a complete absolute response whereas the patient with a poor therapeutic response had no absolute response. The two other patients had bleeding of unknown severity. One patient had a moderate therapeutic response, but both of them had no absolute response.

Discussion:

Two out of four patients receiving treatment in the study by Stoof et al. were moderate patients. DDAVP was given intravenously for a bleeding in the psoas major muscle and for a kidney bleed. Treatment outcome was respectively poor and unknown.¹⁵ Specific bleeding results from de La Fuente et al. could not be retrieved.¹⁹ Ghirardini et al. describe one moderate patient who received DDAVP for dental extraction, which occurred without bleeding.¹⁸ One patient received DDAVP for muscle hematoma, resulting in a prompt cessation of bleeding.¹⁸ Both administrations were subcutaneous and showed no absolute response (incremental 3 and 4 respectively). Warrier et al. described the intra-nasal administration of DDAVP to one moderate patient who received minor oral surgery without any unusual bleeding and no absolute response.²¹

Variable	Missing	
	Total	Percent
Baseline VWF:Act	87	54,7%
Baseline VWF:Ag	53	33,3%
Peak VWF:Act	137	86,2%
Peak VWF:Ag	121	76,1%
Bloodgroup O	95	59,7%
Age at DDAVP response	11	6,9%

Supplemental Table 2. Missing data predictor variables multivariate analyses.

APPENDIX A

RISE consortium.

The investigators and institutions participating in the RISE study are as follows:

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