

REAPPRAISAL OF DESMOPRESSIN FOR MANAGEMENT OF MILD/MODERATE VON WILLEBRAND DISEASE: RESULTS OF A RETROSPECTIVE ANALYSIS IN 127 ITALIAN PATIENTS SUGGEST FURTHER ORGANIZATION OF PROSPECTIVE OBSERVATIONAL STUDIES TO PREDICT DESMOPRESSIN FAILURE WITH IMMEDIATE

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Background. Desmopressin (DDAVP) has been always considered the treatment of choice for patients with mild/moderate von Willebrand disease (VWD), but no standardized criteria have been reported so far to predict benefits/limits of DDAVP in the management of very large cohort of VWD.

Aims. To establish correct indications of DDAVP, we have recently re-analyzed clinical data of patients who received DDAVP treatment at two Italian Centers during the last 15 years (2010-2025)

Methods. The following criteria were required to include cases into this retrospective analysis: age >12 years, bleeding score >5 by BAT, 10-40 U/dL of Platelet-Derived VWF activity (PD-VWF:ACT) and/or VWF collagen binding assay (VWF:CB), trial to determine biological response with assessment of PD-VWF:ACT, VWF:CB, FVIII:C, VWF:AG at baseline and 1-2 hours following DDAVP. Exclusion criteria were known cases with VWD3 and VWD2B, children <12 years, absence or uncompleted data of trial, no patients consent to retrieve their clinical data. DDAVP was given either by subcutaneous injections or intravenously during minor/major surgeries. Efficacy/safety of DDAVP were assessed by statistics after retrieving data from patients charts into EXCEL

files. DDAVP failure was defined when VWF-C were required. Side effects were also registered.

Results. among 153 cases, 127(83%) met inclusion criteria as VWD1(n=78), VWD1C(n=16), VWD2A(n=15), VWD2M(n=18). Biological response was complete, partial and absent in 75%, 23% and 2%. These 127 VWD received DDAVP for bleeds (n=104), oral surgeries (n=33), deliveries (n=12), minor/major surgeries (n=25). Total injections were 652 with median, range/episode during bleeds (2,1-12), oral surgeries (1,1-10), deliveries (3,1-13), minor/major surgeries (3.6,1-16). Efficacy was excellent/good in bleeding (83%), oral surgery (100%), deliveries (87%), minor/major surgeries (82%). Failure was found in VWD1C who required VWF-C after caesarean section and in 5/25 minor/major surgeries [abdominal(n=2), hysterectomy(n=1), tonsillectomy(n=1), orthopedic(n=1)] in VWD2A and VWD1. Side effects(n=16) were mainly minor (flushing, headache, tachycardia) with 2 cases of water retention reported (>12 doses).

Conclusions. DDAVP should be always recommended as first line therapy in responsive mild/moderate VWD patients in bleeding episodes, oral surgery, deliveries and major surgeries. Prospective clinical observations, as those recently proposed by European Investigators, are required to predict DDAVP failure with immediate indication of VWF-C.