The Official Journal of the Italian Society for the Study of Hemostasis and Thrombosis
SISET
Società Italiana per lo Studio dell'Emostasi e della Trombosi
Corso Nazionale SISET
Bergamo | 23-25 ottobre 2025

29TH NATIONAL CONFERENCE OF THE ITALIAN SOCIETY FOR THE STUDY OF HEMOSTASIS AND THROMBOSIS, 2025

ABSTRACT N. PO39

BLEEDING EVENTS IN VON WILLEBRAND DISEASE TYPE 1 YEARLY TREATABLE WITH DESMOPRESSIN.

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Background and Aims: Subcutaneous and intranasal desmopressin is safe and effective in type 1 VWD, and particularly convenient for pediatric use and home treatment. Thus, it can reduce in-hospital admissions and inherent costs, favor adherence and the prompt control of bleeding. Nevertheless, it is often underused or unavailable. We aimed to establish how many patients with VWD type 1 and how many bleeding episodes can potentially benefit from it.

Methods: We reviewed the literature to estimate the global prevalence of symptomatic patients with VWD type 1 and the bleeding incidence in these patients. We adopted the rate of responsiveness to desmopressin in VWD type 1 as recently reported in the metanalysis of Laan et al. (*Blood* 2025;145:1814): 0.89 (confidence interval, CI, 0.83-0.93).

Results: Real world data indicate that a median prevalence of 4.6 per 100,000 inhabitants worldwide of patients with VWD is seeking care in specialized centers, with a mean of 1.73 and min-max range of 0.1-15.1. This huge variability can be explained by the different awareness and diagnosis rates among countries. In these patients the estimated frequency of bleeds was 3.15 events per patient year. Based on the average number of symptomatic VWD type 1 patients, data on bleeding rates and desmopressin response proportion of 0.89 (CI, 0.83-0.93), we estimated that at least 84,000 people with type 1 in the world have 263,000 yearly bleeding events; of these 220,000 events are represented by heavy menstrual bleeding in 24,500 women in fertile age (15-49 years). Overall, VWD type 1 patients would successfully be treated with desmopressin for at least 234,000 yearly events (CI: 218,000-244,500). These estimates have not included desmopressin use for surgery, childbirth, or the preemptive treatment in case of trauma or for bleeding prophylaxis.

Conclusions: Desmopressin is an important therapeutic tool for quite a lot of patients with type 1 von Willebrand disease. The number of cases with VWD type 1 included in our estimation is lower than that reported by the WFH-AGS 2023, and the number of diagnosed and treated patients will even increase in the future due to the increasing diagnostic accuracy. On the other hand, despite the advantages of desmopressin, some findings indicate its suboptimal use, so that patients with potential or confirmed adequate response to desmopressin have been treated with factor concentrates. Due to the progressive unavailability of the more appropriate formulations for subcutaneous and intranasal use of this drug due to the discontinuation of its production, this phenomenon is likely to worsen.

In conclusion, with a higher availability of intranasal and subcutaneous desmopressin a substantial number of bleeding events in VWD type 1 can be treated successfully, safely, and economically also in low-income countries, thus reducing the use of costly replacement therapies.

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