

ITALIAN RESOURCE USE IN MILD HEMOPHILIA A AND TYPE 1 VON WILLEBRAND DISEASE

A. Gringeri^{1,2}, G.C. Castaman³

¹Fondazione Charta, Milano, Italy; ²Centro di Sanità Solidale, Lucca, Italy; ³Centre for Bleeding Disorders and Coagulation, Careggi University Hospital, Firenze, Italy

Introduction. Hemophilia A and von Willebrand disease (VWD) are the most common inherited bleeding disorders, resulting from low or dysfunctional factor VIII (FVIII) and von Willebrand factor (VWF), respectively. The purpose of the following analysis is to quantify the potential savings through desmopressin (DDAVP) use in terms of pharmaceutical costs alone (costs for concentrates), specifically for the treatment of those forms of von Willebrand disease (VWD) and hemophilia with high rates of DDAVP responsiveness, namely type 1 VWD and mild Hemophilia A. In fact, treatment of these disorders has relied mostly on desmopressin, which use however has been significantly reduced over the recent years due to reduced production and supply.

Methods. This analysis is based on official 2023 data of the Italian Registry of Congenital Bleeding Disorders (Registry), published in 2025 by the Italian National Blood Center (CN-S) and by the National Reports on Medicines use in Italy on medicine expenditure referring to 2024 of the Italian Agency of Medicine (AIFA) OsMed.

Results. In Italy, CNS reported an annual consumption in 2023 of 536 million IU of FVIII concentrates, which includes toll fraction concentrates. A total expenditure in 2024 of 310 million € corresponding to 497 million IU has been re-

ported by AIFA. Of these, 83% are recombinant FVIII products used by patients with hemophilia A only. The remaining 17% is represented by pure plasma FVIII (8%), FVIII/VWF products (8%) and pure VWF products (1%). Mild hemophilia A patient treatment represents 7.4% of overall factor consumption, predominantly recombinant products (97%), with an estimated overall expenditure of 22.9 million €. Type 1 VWD patient treatment includes 3% of overall factor consumption, that is 29% of plasma-derived products with an estimated expenditure of 6.7 million €.

Discussion. Current care of mild hemophilia A and type 1 VWD (4,800 estimated patients in Italy) relies largely on the use of intravenous concentrates, with an estimated annual expenditure of 29.6 million € to cover the consumption of 61 million IU (with an average cost of 0.48 €/IU). Improved access to subcutaneous and intranasal desmopressin (DDAVP) and guideline-concordant first-line use in eligible patients could optimize management in responders (reported response rate >80%) and reduce concentrate use and related increased costs. Moreover, it can reduce other direct and indirect costs due to the need to access outpatient clinic for IV infusion and potentially improve quality of life through home treatment.