

## Prospective observational study on DDAVP in VWD: biological response versus clinical efficacy

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Simona Maria Siboni obtained her Medical Degree in 1997 and completed her fellowship in hematology in 2004. In 1998, she began her clinical and scientific education at the Department of Internal Medicine, University of Milan, chaired by Professor Mannucci. She was involved in the care of patients with von Willebrand disease (VWD) at the Angelo Bianchi Bonomi Hemophilia Thrombosis Center under the supervision of Professor Federici and participated in a prospective trial on the use of desmopressin (1-deamino-8-D-arginine vasopressin; DDAVP) in severe forms of VWD (Federici et al, Blood 2003, published online).

DDAVP is a synthetic analogue of vasopressin, originally developed for the treatment of diabetes insipidus. DDAVP increases FVIII:C and VWF plasma concentrations without side effects when administered to healthy volunteers or patients with mild hemophilia A and VWD. Despite the fact that DDAVP has been widely used in clinical practice since 1977,

questions remain about its efficacy in repeated bleeding episodes and during surgery. No prospective studies have correlated biological response and pharmacokinetic (PK) analysis with clinical efficacy in different types of VWD.

The aim of this project is to correlate the biological response (and PK) with the clinical efficacy of DDAVP in type 1 and 2 VWD patients followed for at least two years in a worldwide, prospective observational study organized on behalf of the ISTH-SSC on VWF ([www.ddavp-in-vwd.com](http://www.ddavp-in-vwd.com)). Simona Maria Siboni is applying for this Clinical Scholarship Award to follow VWD patients at the Angelo Bianchi Bonomi Center and to evaluate biological response and clinical efficacy data from VWD cases from other Hemophilia Centers on the database.

Inclusion criteria of the study: VWD patients with type 1 and 2 disease, aged between 5 and 70 years. Exclusion criteria: a) acquired von Willebrand syndrome; b) additional congenital and acquired defects of platelet function; c) use of anti-inflammatory drugs affecting platelet function; d) previous history of seizures in the family; e) recent serious cardiovascular episodes. Biological response and PK of FVIII/VWF activities will be evaluated with predefined criteria following an infusion trial of DDAVP (intravenous injection of 0.3 ug/Kg, diluted in 50-100 ml saline, over 30 min) in patients without bleeding or off-surgery. Clinical efficacy will be evaluated following predefined criteria. Once enrolled in the study, all patients

will be followed prospectively for at least two years for any bleeding episodes and invasive procedures or minor-major surgeries; clinical data with FVIII/VWF activities before and after each DDAVP injection will be reported directly on the online database.

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