

Indication for use of VWF containing concentrates in patients with von Willebrand disease

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INTRODUCTION

- von Willebrand disease (VWD) is the most common inherited bleeding disorder affecting both genders
- treatment of von Willebrand disease (VWD) aims to increase FVIII and VWF activities in order to prevent or stop the bleeding complications
- treatment options are desmopressin (DDAVP) and von Willebrand factor (VWF) containing plasma derived concentrates (VWF concentrates)

METHODS

- single center retrospective study
- from 2000 to 2015
- **inclusion criteria:**
 - all patients with VWD referred to our center
 - VWF:Ag and/or VWF:RCo levels ≤40 U/dL

Definitions:

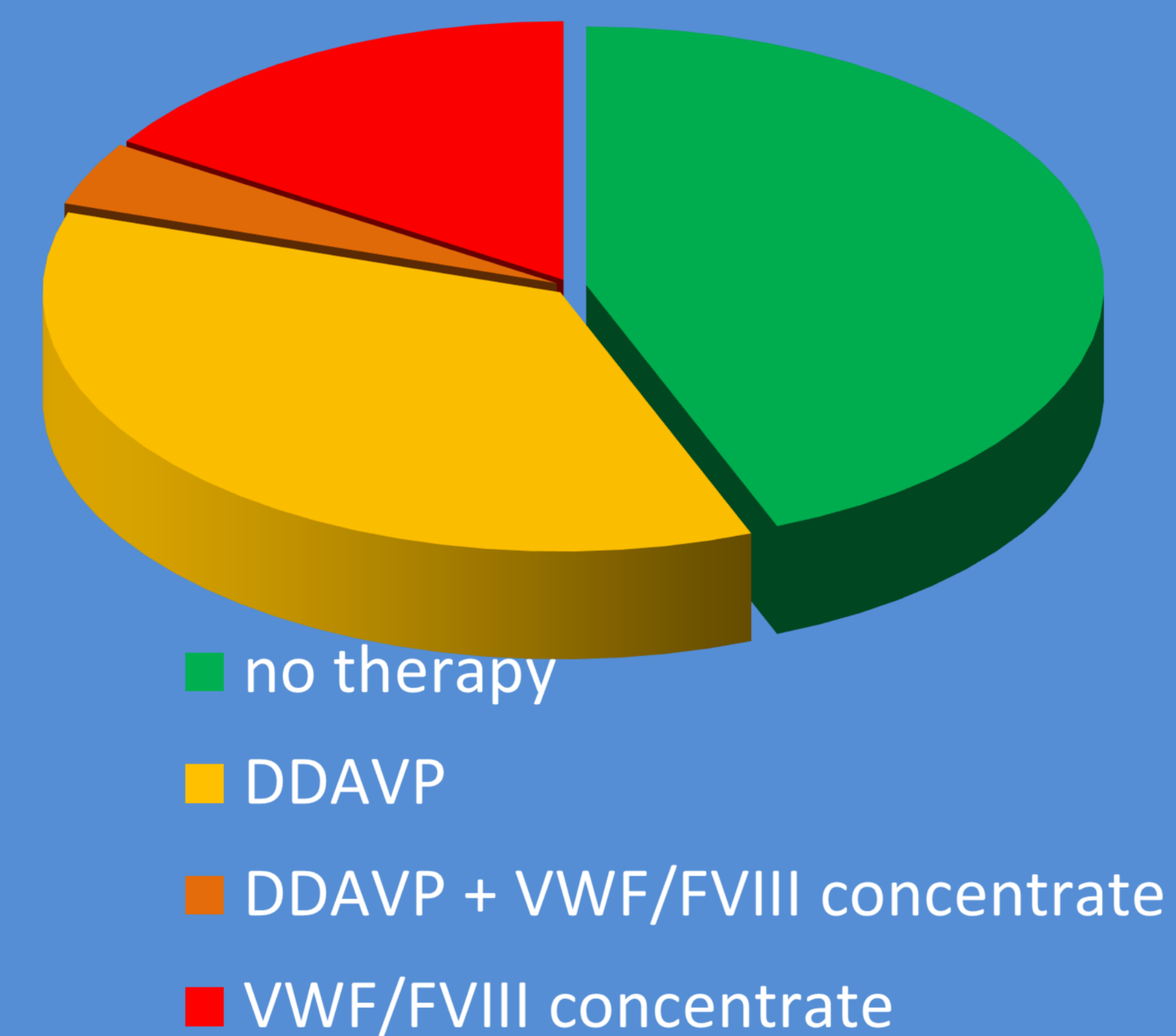
- severe VWD - VWF/Roc <10 IU/dL
- moderate VWD - VWF/Roc 10-30 IU/dL
- mild VWD - VWF/Roc 30-40 IU/dL

Patient characteristics

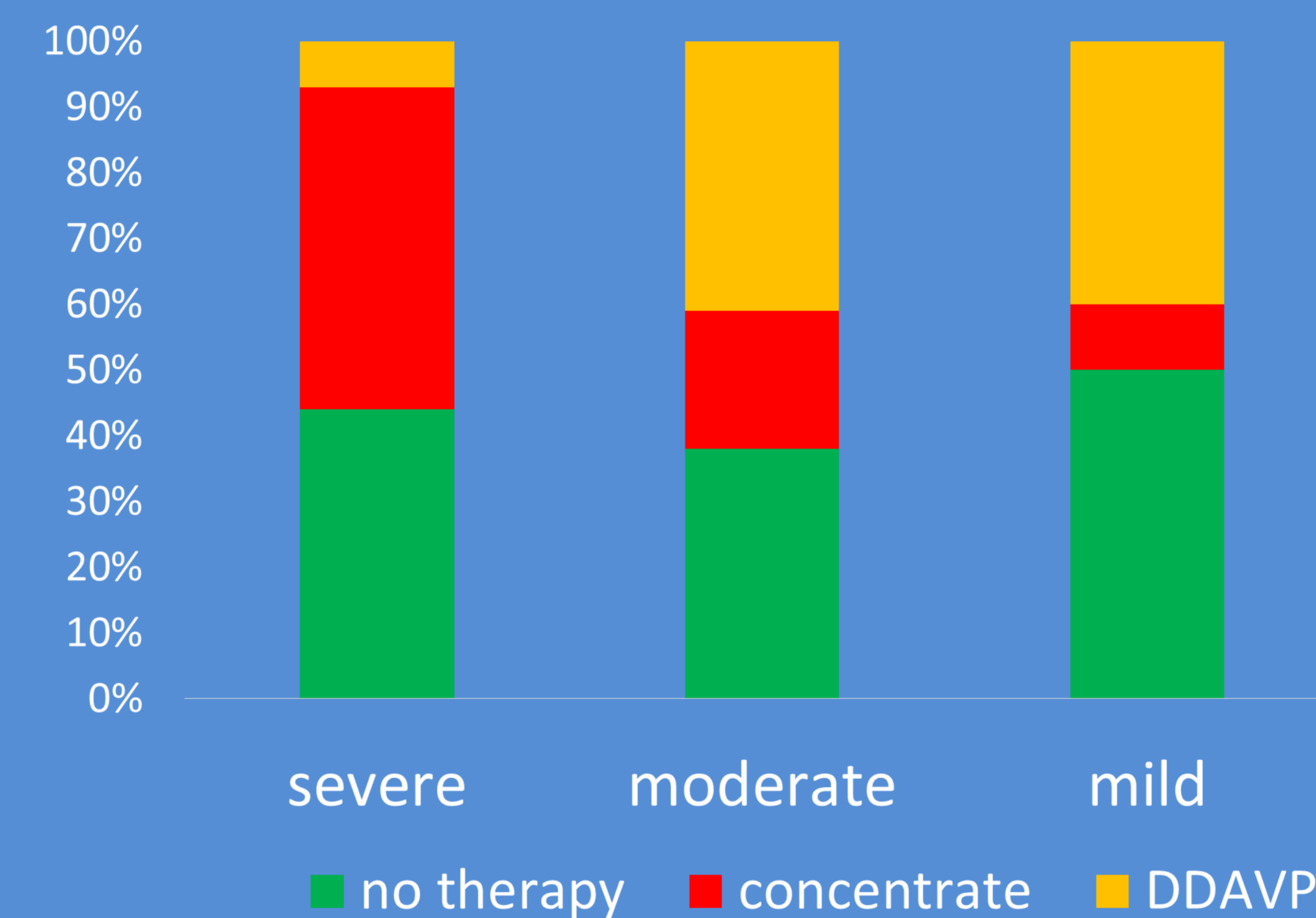
	All types	Type 1	Type 2	Type 3
Total number	174	118	53	3
Age (years), median (range)	33 (3-81)	32 (3-77)	37 (11-81)	38 (29-46)
Gender, m/f	61/113	40/78	19/34	2/1
VWF/RCo, IU/dL				
<10	27 (15%)	7 (6%)	17 (32%)	3 (100%)
10-30	74 (43%)	45 (38%)	29 (55%)	0
30-40	73 (42%)	66 (56%)	7 (13%)	0
FVIII:C, IU/dL				
<20	10 (6%)	3 (3%)	4 (8%)	3 (100%)
20-40	33 (19%)	12 (10%)	21 (40%)	0
>40	131 (75%)	103 (87%)	28 (52%)	0

RESULTS

Choice of therapy in all patients



Choice of therapy according to the severity of VWD



Choice of therapy according to the type of VWD

	DDAVP	VWF/FVIII concentrates
All types	61 (64%)	34 (36%)
Type 1	49 (79%)	13 (21%)
Type 2	11 (38%)	18 (62%)
2A	4 (50%)	4 (50%)
2B	1 (50%)	1 (50%)
2M	6 (33%)	12 (67%)
2N	0	1 (100%)
Type 3	0	3 (100%)

Use of concentrate according to the type of disease and bleeding

Type of disease	Number of patients (%)	
	surgery	spontaneous bleeding
type 1	9 (90%)	2 (20%)
type 2A	4 (100%)	0
type 2B	1 (100%)	0
type 2M	12 (92%)	1 (8%)
type 2N	1 (100%)	0
type 3	2 (67%)	3 (100%)

Reason for choosing VWF/FVIII concentrate

	No of patients (34/174)
DDAVP test unresponsive VWD type 3	7 (all type 2M)
Use of DDAVP contraindicated	5
Delivery	7
GI bleeding	2
Major surgery	5
DDAVP test not done	2
Prophylaxis	4
Unknown	2

AIM: to evaluate the need for specific haemostatic therapy using VWF concentrates in patients with VWD, depending on the type and severity of disease and the bleeding pattern

CONCLUSIONS

- only a limited number of patients with VWD need therapy with VWF concentrates
- bleeds in substantial proportion of patients can be managed with DDAVP and/or antifibrinolytics
- besides type 3 disease where the use of DDAVP is ineffective, the use of VWF concentrates was frequent in type 2 and severe type 1 disease, as well as in severe disease regardless the type
- the absolute indication for VWD concentrate therapy is prophylaxis
- VWD concentrates are rarely used for the treatment of spontaneous bleeds

Indications for haemostatic treatment

Indication	VWF/FVIII concentrate	DDAVP
Total number of patients	32	61
Prophylaxis	3 (100%)	0
Spontaneous bleeds	3 (6%)	45 (94%)
Surgical and invasive procedures	22 (37%)	38 (63%)
Delivery	8 (89%)	1 (11%)

