

The status of care for persons with von Willebrand disease registered within CNHP registry Annual Report 2023

Jan Blatný, Petra Ovesná

on behalf of

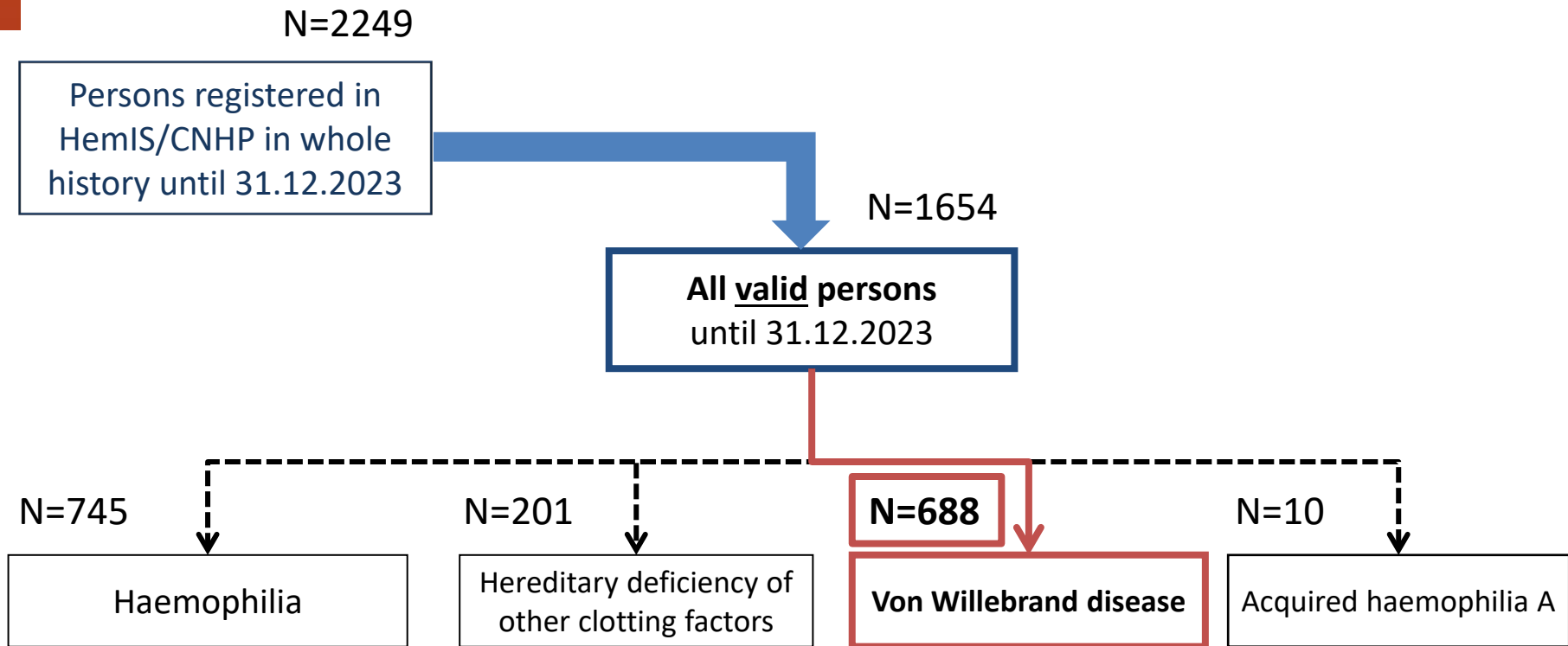
Centres contributing to database
of the CNHP (Czech National Haemophilia Programme)

Export date: April 2, 2024

Persons with Von Willebrand disease



Sample size



Cca 1000 symptomatic vWDs should be in CZ
688 of them are already in CNHP registry

Number of patients in participating centres

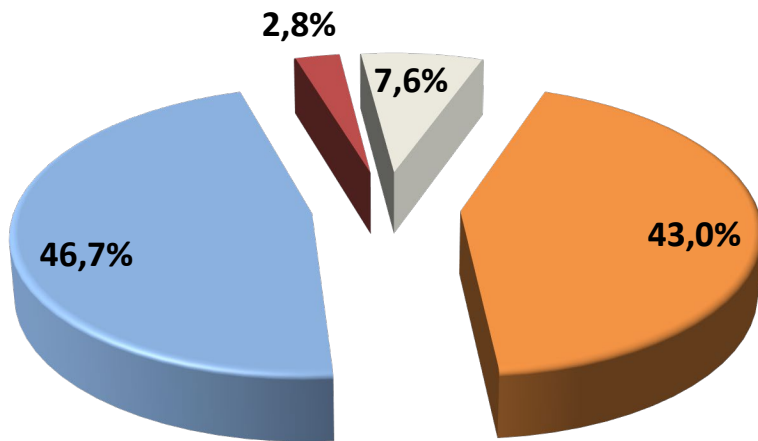
N=688





Paediatric centres	Valid patients	
	N	%
Prague – Dpt. of Pediatric Haematology and Oncology, CUH Motol	52	7.6
Brno – Dpt. of Pediatric Haematology, CUH Brno	29	4.2
Ostrava – Dpt. of Pediatric Medicine, UH Ostrava	28	4.1
Pilsen – Pediatric Dpt., UH Pilsen	25	3.6
Olomouc – Dpt. of Pediatric Medicine, UH Olomouc	11	1.6
Ústí n.L. – Pediatric Dpt. – Haematology, Masaryk Hospital	7	1.0
Hradec Králové – Dpt. of Pediatric Medicine, UH HK	6	0.9

Adult centres	Valid patients	
	N	%
Brno – Dpt. Of Clin Hematol, UH Brno	308	44.8
Pilsen – Dpt. of Biochemistry and Hematology, UH Pilsen – UKBH	81	11.8
Ostrava – Blood centre, UH Ostrava	57	8.3
Liberec – Dpt. Of Clin Hematol, Hospital Liberec	33	4.8
Olomouc – Haemato-Oncology Dpt., UH Olomouc	28	4.1
Ústí n.L. – Dpt. Of Clin Hematol, Masaryk Hospital	23	3.3

Type of Von Willebrand's disease

N=688



		Patients	
Type of disease		N	%
	Type 1*	296	43.0
	Type 2	321	46.7
	Type 2A	130	18.9
	Type 2B	22	3.2
	Type 2M	50	7.3
	Type 2N	35	5.1
	Type 2 (not specified)	84	12.2
	Type 3	19	2.8
	Type not determined / unfilled	52	7.6
Total		688	100.0

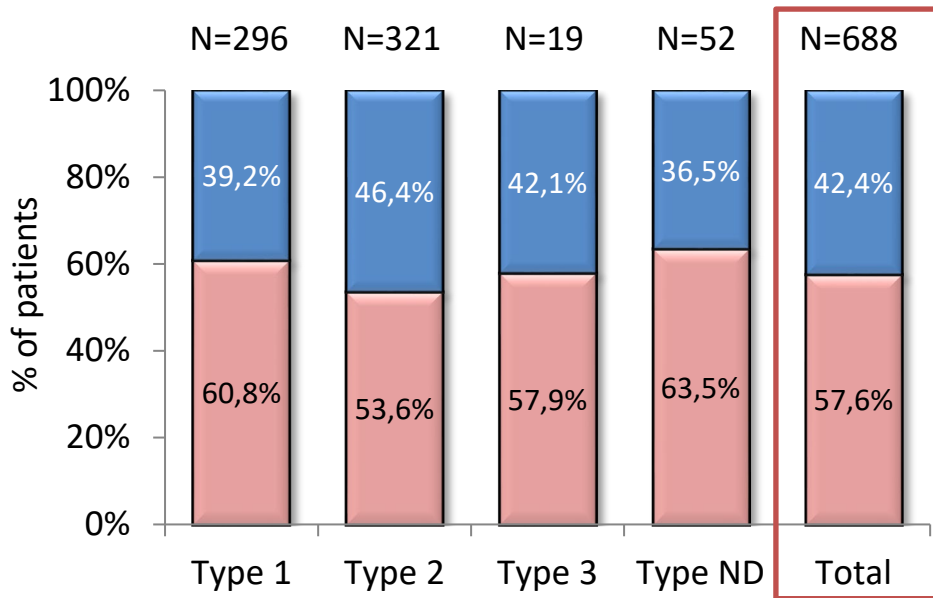
* of them 7 patients with type 1/2N

Sex and current age of patients

N=688

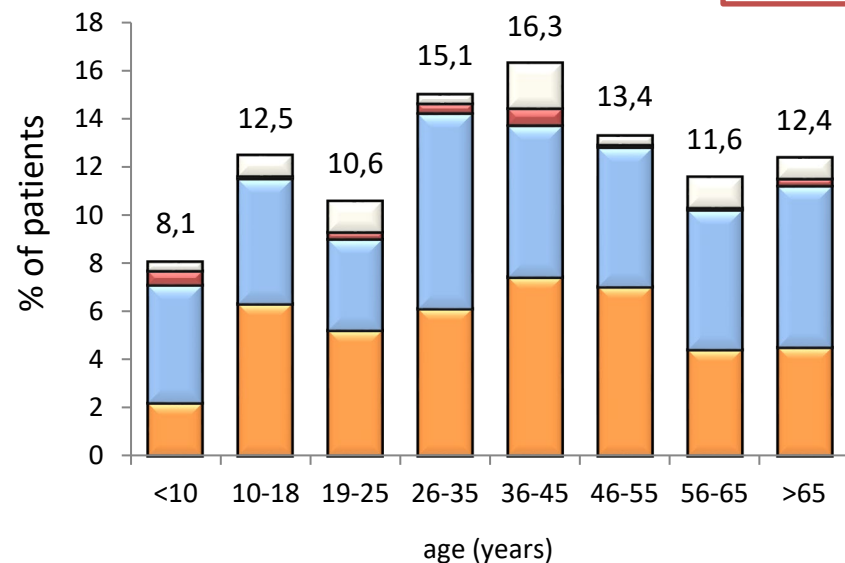
Sex

- Men (N=292)
- Women (N=396)



Current age*

	Type 1	Type 2	Type 3	Type ND	Total
N	296	321	19	52	688
Mean	38.1	39.1	32.4	39.6	38.5
Median	38	37	30	38.5	38
min - max	3-86	1-87	1-72	2-85	1-87

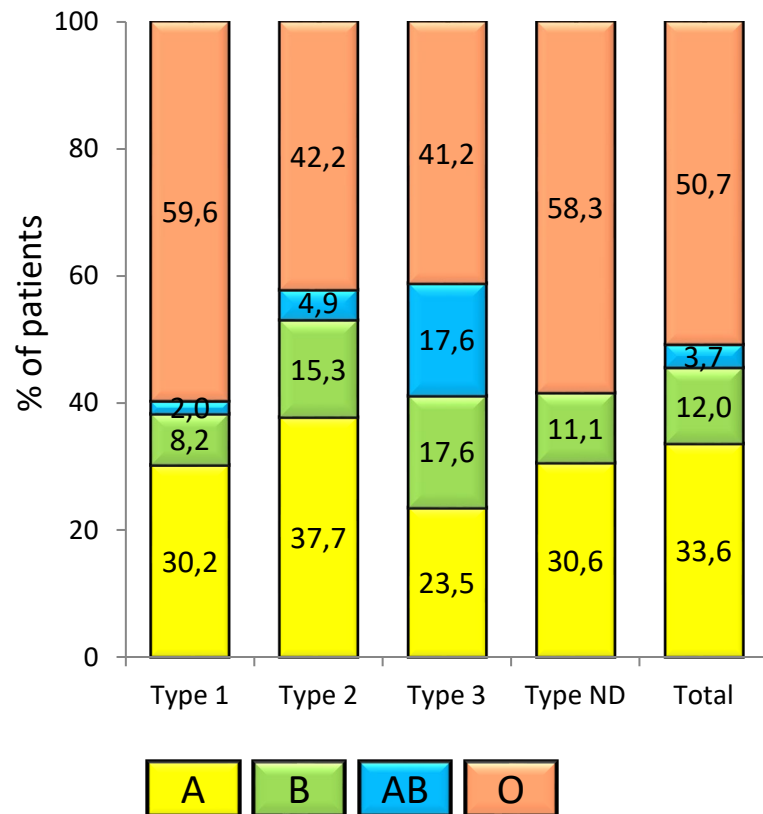


Type ND = not determined or unfilled

* age reached in year 2023

Blood group

N=688

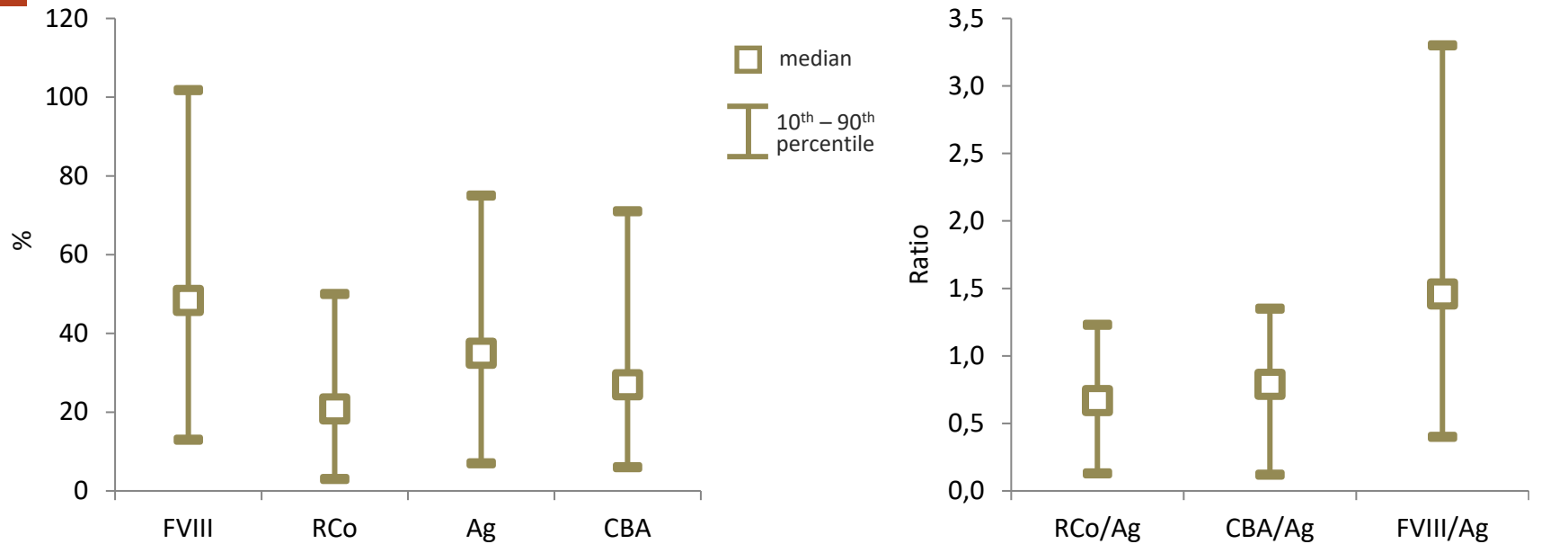


%¹ - % of total N
 %² - % of valid N

Blood group	A	B	AB	O	ND	Valid N	Total N
Type 1	N	74	20	5	146	51	245
	%¹	25.0	6.8	1.7	49.3	17.2	100.0
	%²	30.2	8.2	2.0	59.6		100.0
Type 2	N	101	41	13	113	53	268
	%¹	31.5	12.8	4.0	35.2	16.5	100.0
	%²	37.7	15.3	4.9	42.2		100.0
Type 3	N	4	3	3	7	2	17
	%¹	21.1	15.8	15.8	36.8	10.5	100.0
	%²	23.5	17.6	17.6	41.2		100.0
Type ND	N	11	4	0	21	16	36
	%¹	21.2	7.7	0.0	40.4	30.8	100.0
	%²	30.6	11.1	0.0	58.3		100.0
Total	N	190	68	21	287	122	566
	%¹	27.6	9.9	3.1	41.7	17.7	100.0
	%²	33.6	12.0	3.7	50.7		100.0

Factor levels

N=688

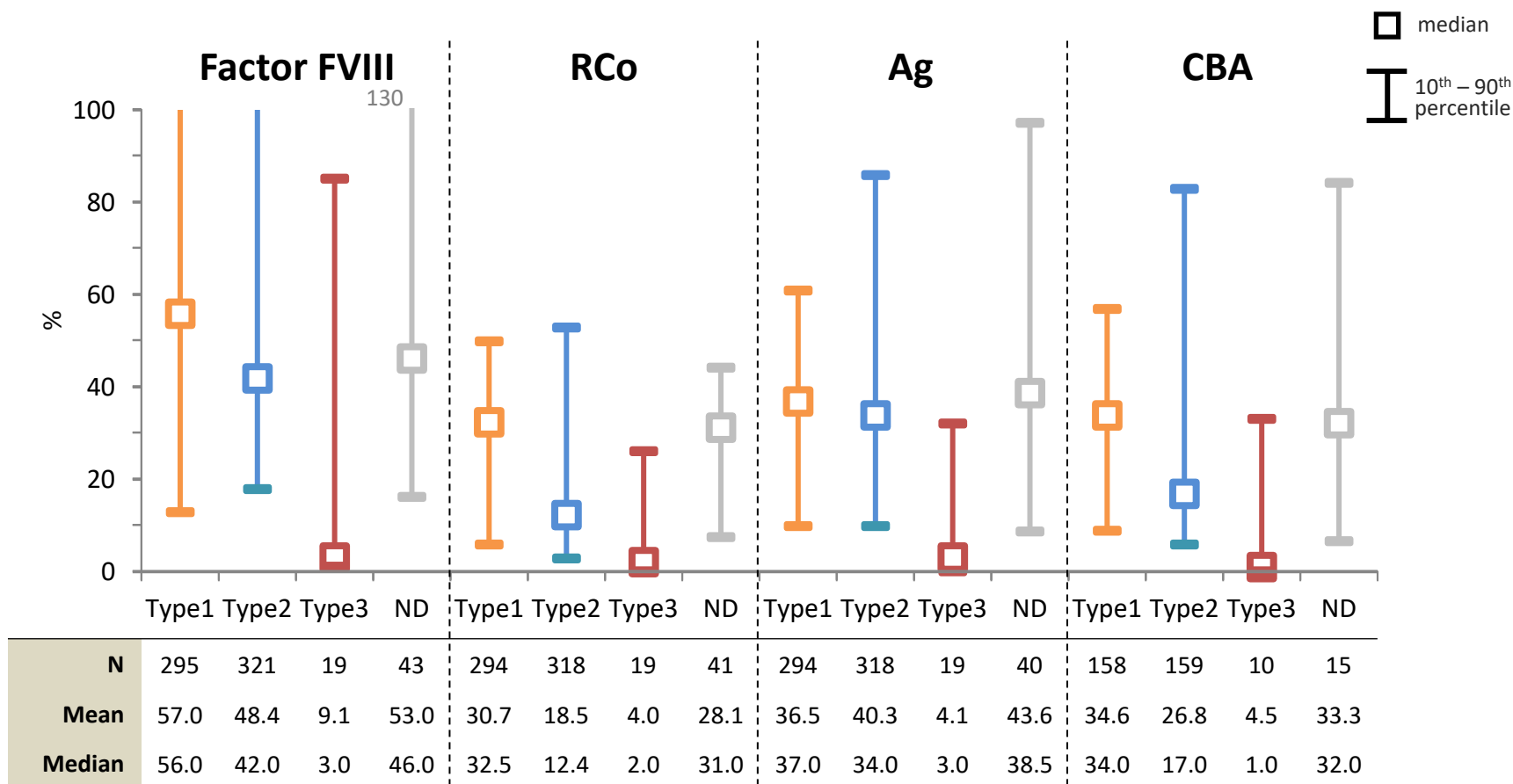


Factor FVIII*	RCo	Ag	CBA		RCo/Ag	CBA/Ag	FVIII/Ag
678	672	671	342	N	663	341	667
51.3	24.0	37.8	30.1	Mean	0.8	0.8	1.8
48.4 (1–232)	20.85 (0–113)	35 (0–261)	27 (0–127)	Median (min – max)	0.67 (0–38)	0.79 (0–3)	1.46 (0.08–106)

* Factor FVIII was assessed by coagulation method in 697 patients, by chromogenic method in 20 patients and method is missing in 29 patients.

Factor levels according to type of VWD, part 1

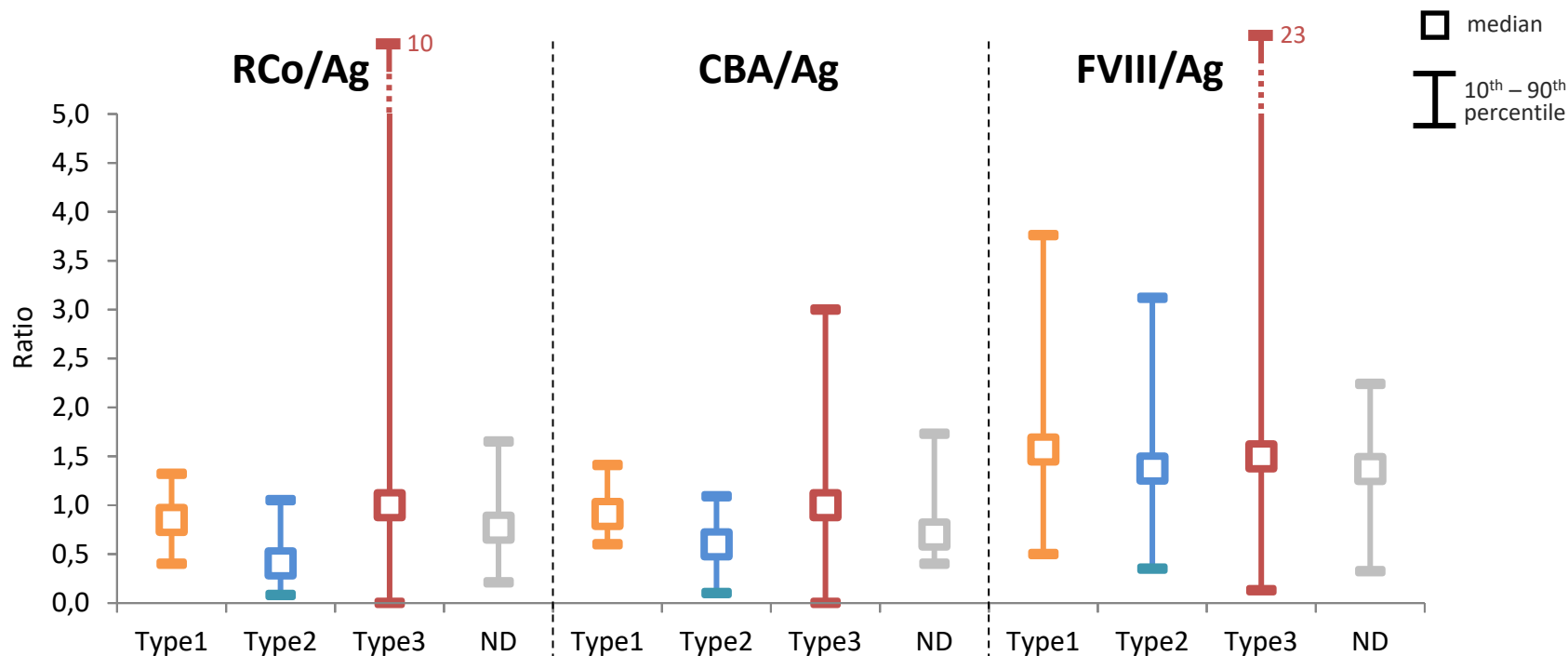
N=688



Type ND = not determined or unfilled

Factor levels according to type of VWD, part 2

N=688



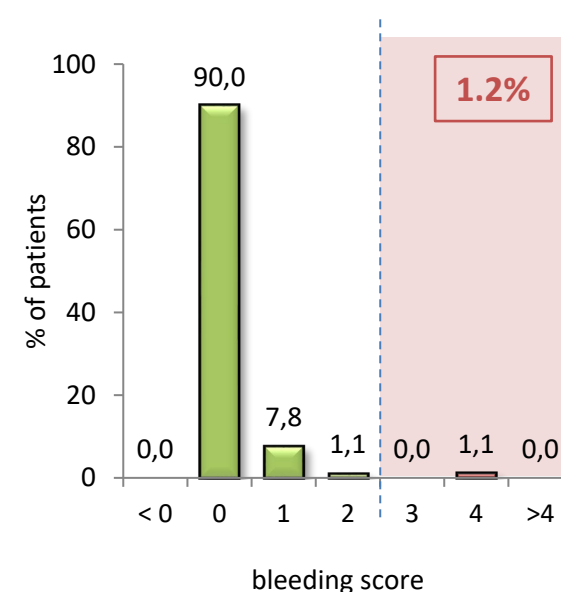
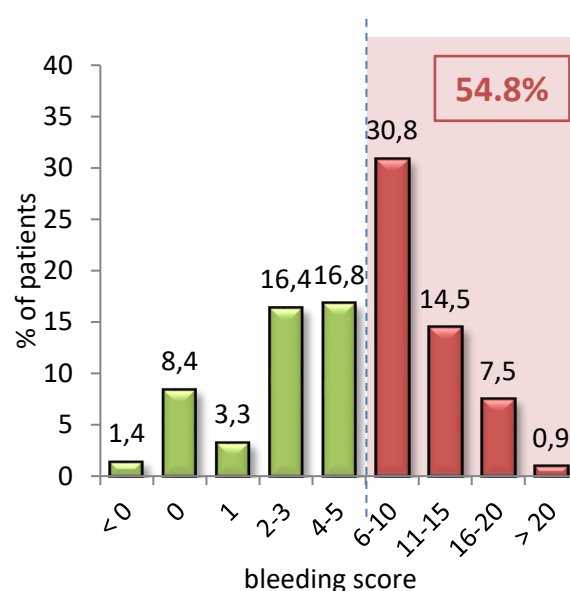
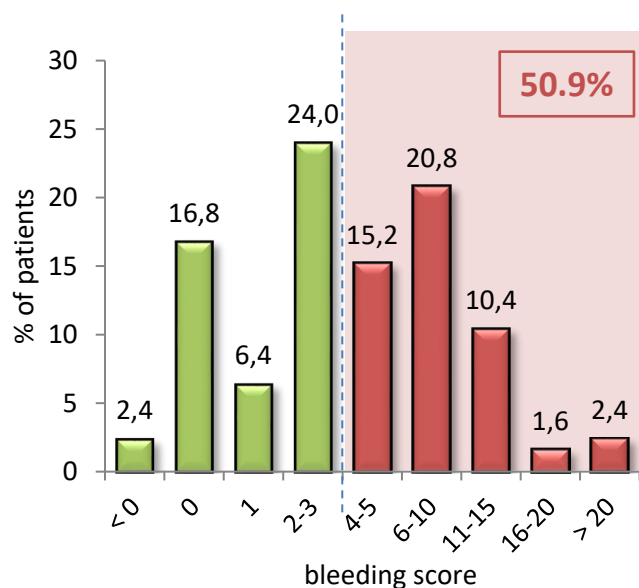
	Type1	Type2	Type3	ND	Type1	Type2	Type3	ND	Type1	Type2	Type3	ND
N	293	314	17	39	157	159	10	15	294	316	17	40
Mean	1.03	0.47	1.41	0.76	0.96	0.58	0.90	0.82	2.18	1.52	3.33	1.36
Median	0.85	0.41	1.00	0.77	0.91	0.60	1.00	0.70	1.57	1.38	1.50	1.37

Type ND = not determined or unfilled

Bleeding score¹ according to sex and age

N=429²

	Adult men	Adult women	Children
N	125	214	90
Mean	5.0	6.9	0.1
Median (min - max)	4 (-2 - 25)	6 (-2 - 24)	0 (0 - 4)



¹ Adult and Pediatric Vincenza VWD Bleeding Questionnaire and Scoring System

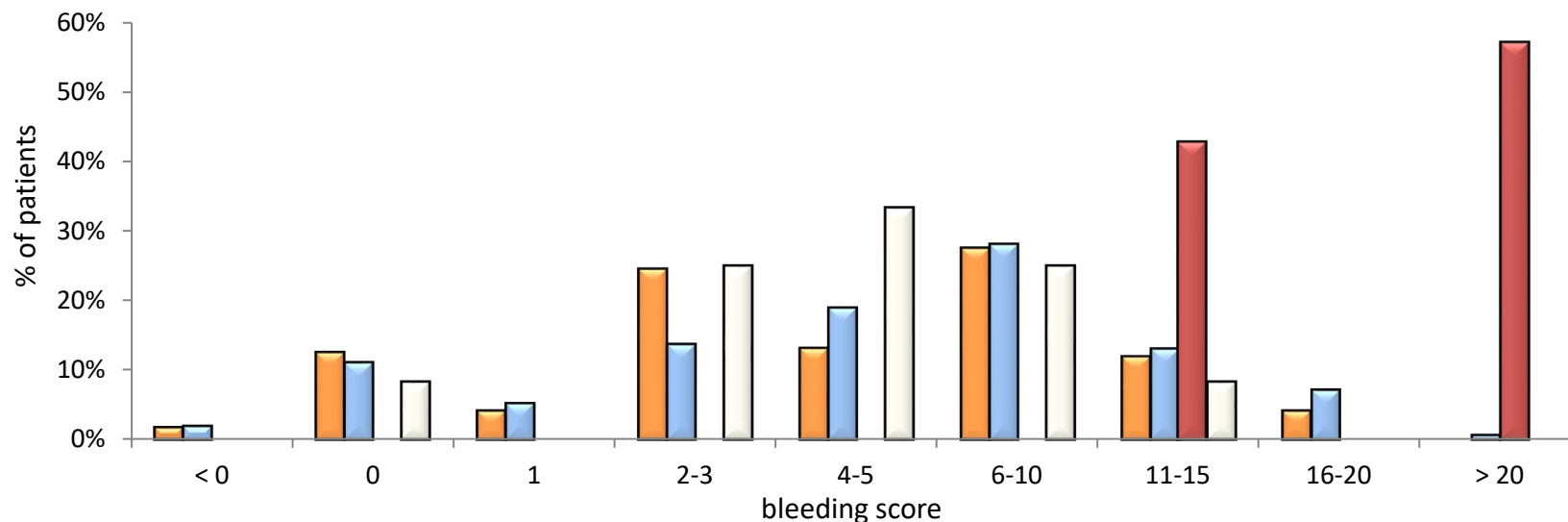
² Missing information on bleeding score in 259 patients.

Bleeding score¹ in adults according to type of disease

N=339²

Bleeding score

	Type 1	Type 2	Type 3	Type ND	Total
N total	238	251	14	43	546
N valid	167	153	7	12	339
Mean	5.5	6.5	18.9	5.4	6.2
Median	5.0	5.0	21.0	4.5	5.0
min - max	-2-20	-1-22	13-25	0-14	-2-25



¹ Adult and Pediatric Vincenza VWD Bleeding Questionnaire and Scoring System

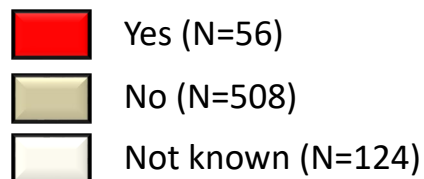
² Adult patients with non-missing information on bleeding score.

Type ND = not determined or unfilled

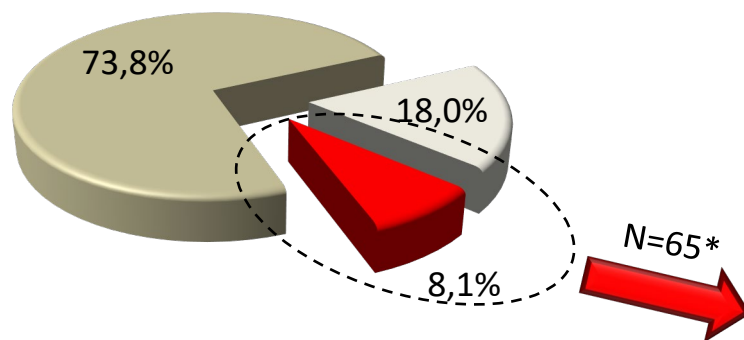
Concomitant diseases

N=688

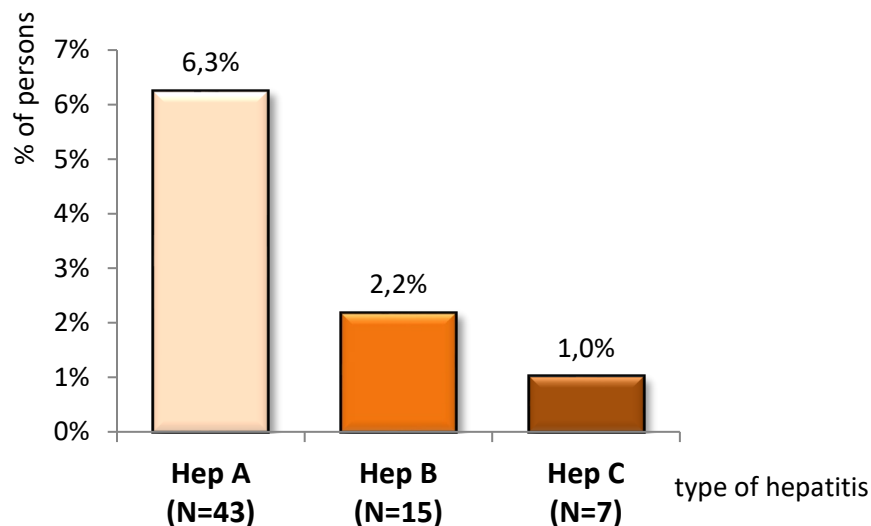
Experienced hepatitis



None of the patients is HIV positive.



*Total of 65 cases of hepatitis in 56 persons. One person may have more types of hepatitis recorded.



Annual bleeding rate (ABR) and location of bleeds treated with factor concentrate

	Type 1	Type 2	Type 3	Type ND	Total	N=688
ABR						
N valid	296	321	19	52	688	
Mean	0.04	0.11	1.47	0.00	0.11	
Median	0	0	0	0	0	
min-max	0-8	0-9	0-19	0-0	0-19	
SUM	11	34	28	0	73	
<hr/>						
Location of bleeds						
N_p/N_B^1						
Joints	2/9	1/1	1/3	0/-	4/13	
Epistaxes	1/1	8/22	2/20	0/-	11/43	
Urogenital tract	-/-	-/-	-/-	-/-	-/-	
GIT	-/-	-/-	-/-	-/-	-/-	
Subcutaneous	0/-	1/1	2/2	0/-	3/3	
Muscles	0/-	1/1	0/-	0/-	1/1	
CNS	-/-	-/-	-/-	-/-	-/-	
Metrorrhagia	0/-	1/1	1/1	0/-	2/2	
Other	1/1	5/6	2/2	0/-	8/9	
SUM	4/11	17/32	8/28	0/-	29/71	

¹ N_p = number of patients with bleed;

N_B = total number of bleeds treated with substitution of VWF

Type ND = not determined or unfilled

Consumption of VWF concentrates in year 2023

N=688

	Number of patients treated with substitution of VWF total (type1/type2/type3/typeND)	Total annual consumption ¹ (IU)	Average annual consumption ¹ per treated patient (IU)
Haemate P	65 (18/38/7/2)	1 711 310	26 327.8
<i>of them on prophylaxis*</i>	<i>5 (0/1/4/0)</i>	<i>1 004 000</i>	<i>200 800.0</i>
Wilate	21 (10/7/4/0)	618 980	29 475.2
<i>of them on prophylaxis*</i>	<i>4 (0/0/4/0)</i>	<i>488 480</i>	<i>122 120.0</i>
Fanhdi	4 (2/2/0/0)	45 800	11 450.0
<i>of them on prophylaxis*</i>	<i>0 (0/0/0/0)</i>	<i>0</i>	<i>0.0</i>
Total	90 (30/47/11/2)	2 376 090	26 401.0
<i>of them on prophylaxis*</i>	<i>9 (0/1/8/0)</i>	<i>1 492 480</i>	<i>165 831.1</i>
Total - type 1	30 (30/0/0/0)	224 010	<u>7 467.0</u>
Total - type 2	47 (0/47/0/0)	418 600	<u>8 906.4</u>
Total - type 3	11 (0/0/11/0)	1 730 980	<u>157 361.8</u>
Total - type ND	2 (0/0/0/2)	2 500	<u>1 250.0</u>

* permanent prophylaxis

Type ND = not determined or unfilled

¹ The consumption of Haemate P, Wilate and Fanhdi is given in FVIII units.

Consumption of other drugs in year 2023

N=688

	Number of patients treated with other drugs total (type1/type2/type3/typeND)	Total annual consumption	Average annual consumption per <u>treated</u> patient
DDAVP (µg)	2 (1/1/0/0)	1 512	756.0
<i>of them on prophylaxis*</i>	<i>0 (0/0/0/0)</i>	<i>0</i>	<i>0.0</i>

* *permanent prophylaxis*

Type ND = not determined or unfilled

Patients on permanent prophylaxis in detail

N=8

No	Centre	Sex	Age group	Type of VWD	Type of prophylaxis in 2023	Treatment in 2023	No of applications per week	Total consumption (IU*)	ABR in 2023
1	Praha	Male	Child	3	Permanent	Wilate	3	199 800	19
2	Praha	Female	Child	3	Permanent	Wilate	3	56 000	0
3	Ostrava	Male	Child	3	Permanent	Wilate	2	65 680	4
4	Brno	Female	Adult	3	Permanent	Haemate P Wilate	2.33	198 000 167 000	1
5	Brno	Female	Adult	3	Permanent	Haemate P	2.33	377 000	0
6	Brno	Female	Adult	3	Permanent	Haemate P	1.6	240 000	1
7	Brno	Male	Adult	3	Permanent	Haemate P	2	182 000	0
8	Liberec	Male	Adult	2	Permanent	Haemate P	2	7 000	0

* The consumption is given in FVIII units.