

# The status of care for persons with von Willebrand disease registered within CNHP registry Annual Report 2018

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on behalf of

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

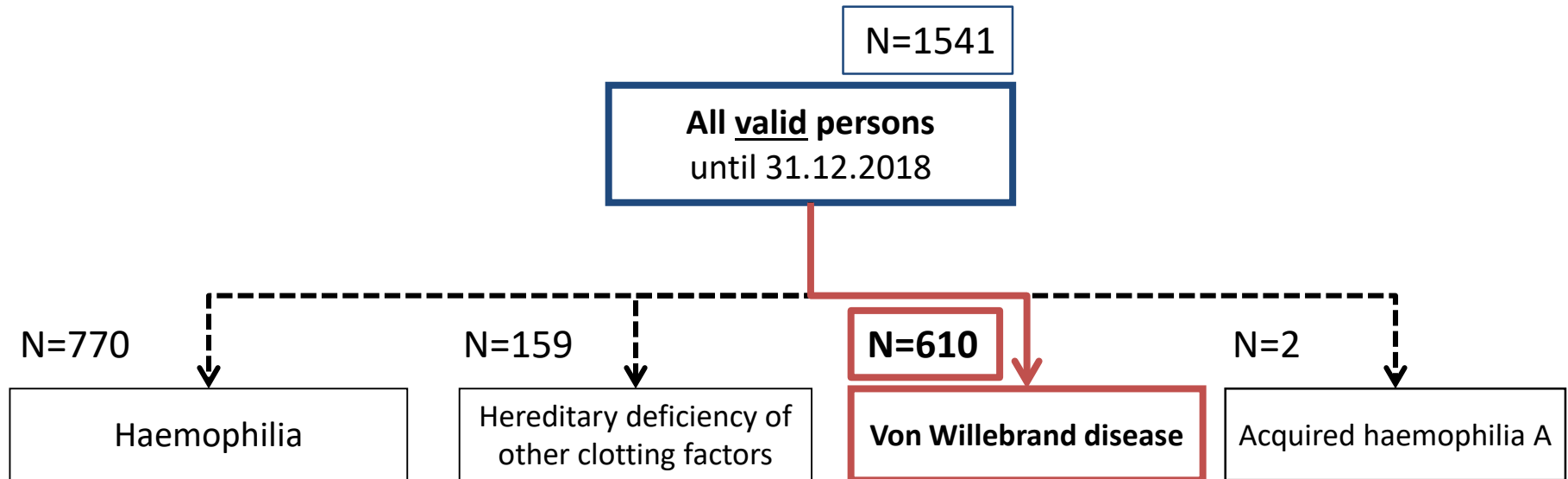
*Export date: April 3, 2019*



# Persons with Von Willebrand disease



# Sample size



Cca 1000 symptomatic vWD should be in CZ  
610 of them are already in CNHP registry

# Number of patients in participating centres

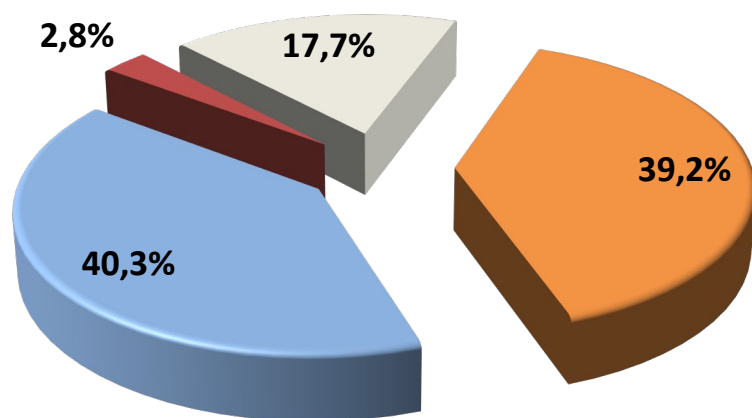
N=610





Paediatric centres	Valid patients	
	N	%
<b>Prague</b> – Dpt. of Pediatric Haematology and Oncology, CUH Motol	28	4.6
<b>Pilsen</b> – Pediatric Dpt., UH Pilsen	25	4.1
<b>Brno</b> – Dpt. of Pediatric Haematology, CUH Brno	22	3.6
<b>Hradec Králové</b> – Dpt. of Pediatric Medicine, UH HK	18	3.0
<b>Ostrava</b> – Dpt. of Pediatric Medicine, UH Ostrava	16	2.6
<b>Ústí n.L.</b> – Pediatric Dpt. – Haematology, Masaryk Hospital	14	2.3
<b>Olomouc</b> – Dpt. of Pediatric Medicine, UH Olomouc	1	0.2

Adult centres	Valid patients	
	N	%
<b>Brno</b> – Dpt. Of Clin Hematol, UH Brno	288	47.2
<b>Pilsen</b> – Dpt. of Biochemistry and Hematology, UH Pilsen – UKBH	74	12.1
<b>Ostrava</b> – Blood centre, UH Ostrava	70	11.5
<b>Liberec</b> – Dpt. Of Clin Hematol, Hospital Liberec	27	4.4
<b>Olomouc</b> – Haemato-Oncology Dpt., UH Olomouc	14	2.3
<b>Ústí n.L.</b> – Dpt. Of Clin Hematol, Masaryk Hospital	10	1.6
<b>Hradec Králové</b> – IV. Internal and Hematology Dpt., UH HK	2	0.3
<b>České Budějovice</b> – Dpt. Of Clin Hematol, Hospital CB	1	0.2

# Type of Von Willebrand disease

N=610



		Patients	
Type of disease		N	%
	Type 1*	239	39.2
	Type 2	246	40.3
	Type 2A	104	17.0
	Type 2B	19	3.1
	Type 2M	42	6.9
	Type 2N	21	3.4
	Type 2 (not specified)	60	9.8
	Type 3	17	2.8
	Type not determined / unfilled	108	17.7
Total		610	100.0

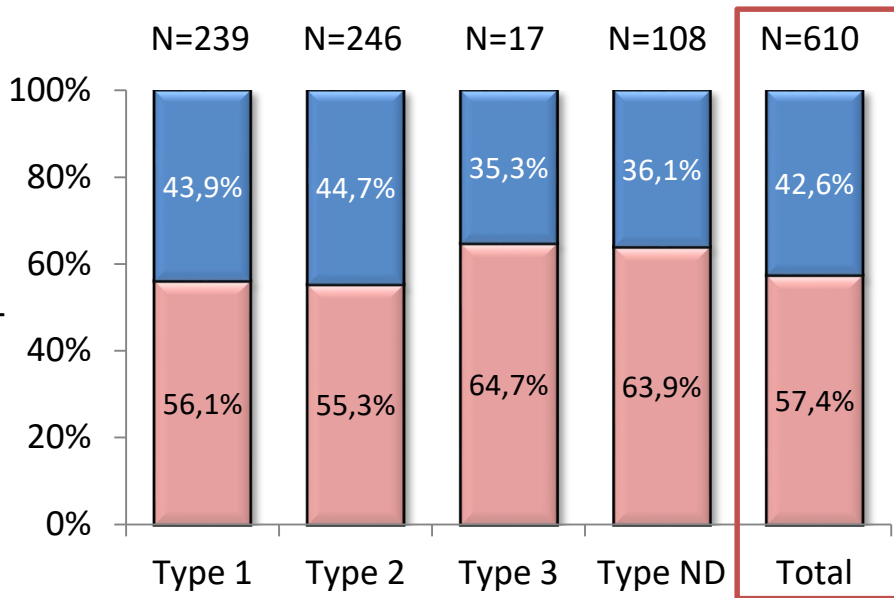
\* of them 6 patients with type 1/2N

# Sex and current age of patients

**N=610**

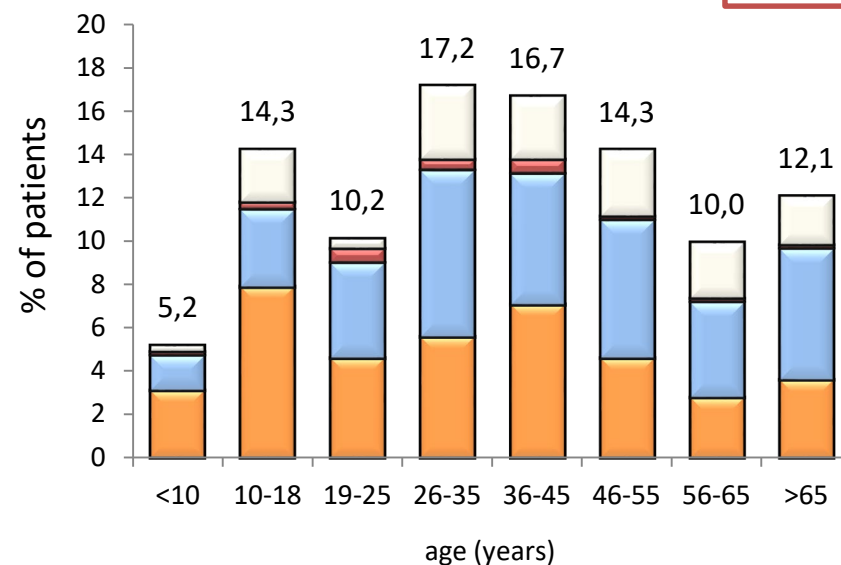
## Sex

■ Men (N=260)  
■ Women (N=350)



## Current age\*

	Type 1	Type 2	Type 3	Type ND	Total
<b>N</b>	239	246	17	108	<b>610</b>
<b>Mean</b>	34.9	41.7	33.5	43.0	<b>39.0</b>
<b>Median</b>	34	41	32	43.5	<b>39</b>
<b>min - max</b>	3 - 90	2 - 85	2 - 67	5 - 80	<b>2 - 90</b>

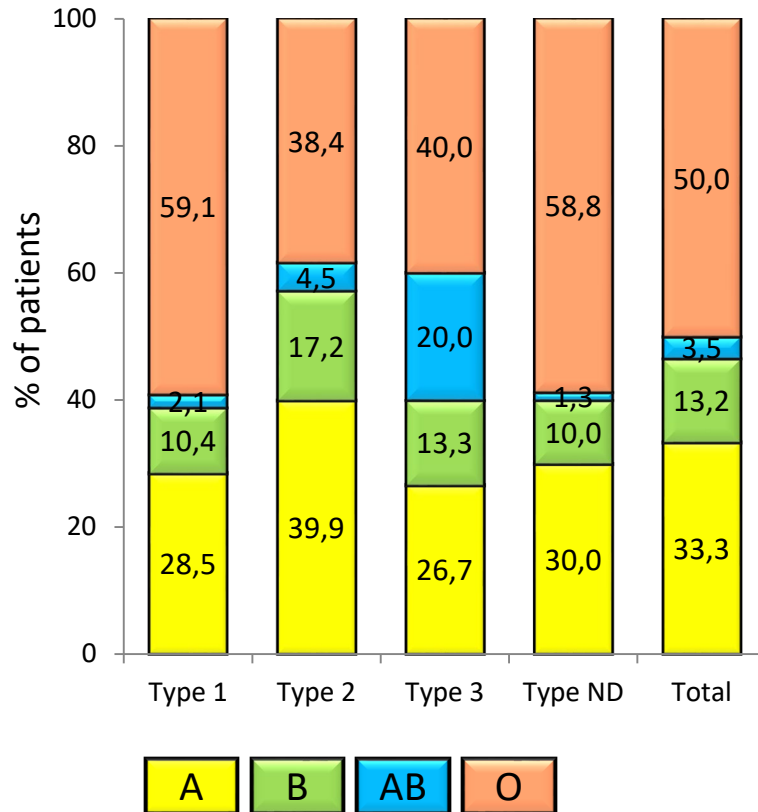


Type ND = not determined or unfilled

\* age reached in year 2018

# Blood group

N=610

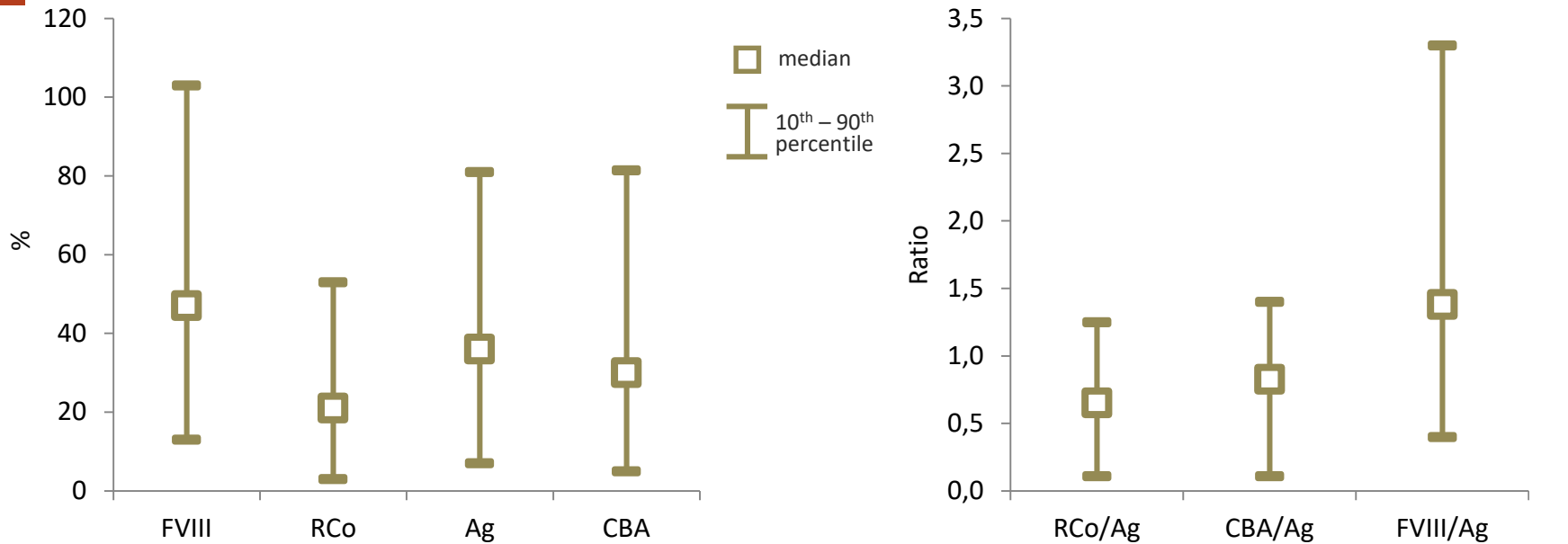


%<sup>1</sup> - % of total N  
 %<sup>2</sup> - % of valid N

Blood group	A	B	AB	O	ND	Valid N	Total N
<b>Type 1</b>	<i>N</i>	55	20	4	114	46	193
	% <sup>1</sup>	23.0	8.4	1.7	47.7	19.2	100.0
	% <sup>2</sup>	28.5	10.4	2.1	59.1		100.0
<b>Type 2</b>	<i>N</i>	79	34	9	76	48	198
	% <sup>1</sup>	32.1	13.8	3.7	30.9	19.5	100.0
	% <sup>2</sup>	39.9	17.2	4.5	38.4		100.0
<b>Type 3</b>	<i>N</i>	4	2	3	6	2	15
	% <sup>1</sup>	23.5	11.8	17.6	35.3	11.8	100.0
	% <sup>2</sup>	26.7	13.3	20.0	40.0		100.0
<b>Type ND</b>	<i>N</i>	24	8	1	47	28	80
	% <sup>1</sup>	22.2	7.4	0.9	43.5	25.9	100.0
	% <sup>2</sup>	30.0	10.0	1.3	58.8		100.0
<b>Total</b>	<i>N</i>	162	64	17	243	124	486
	% <sup>1</sup>	26.6	10.5	2.8	39.8	20.3	100.0
	% <sup>2</sup>	33.3	13.2	3.5	50.0		100.0

# Factor levels

N=610



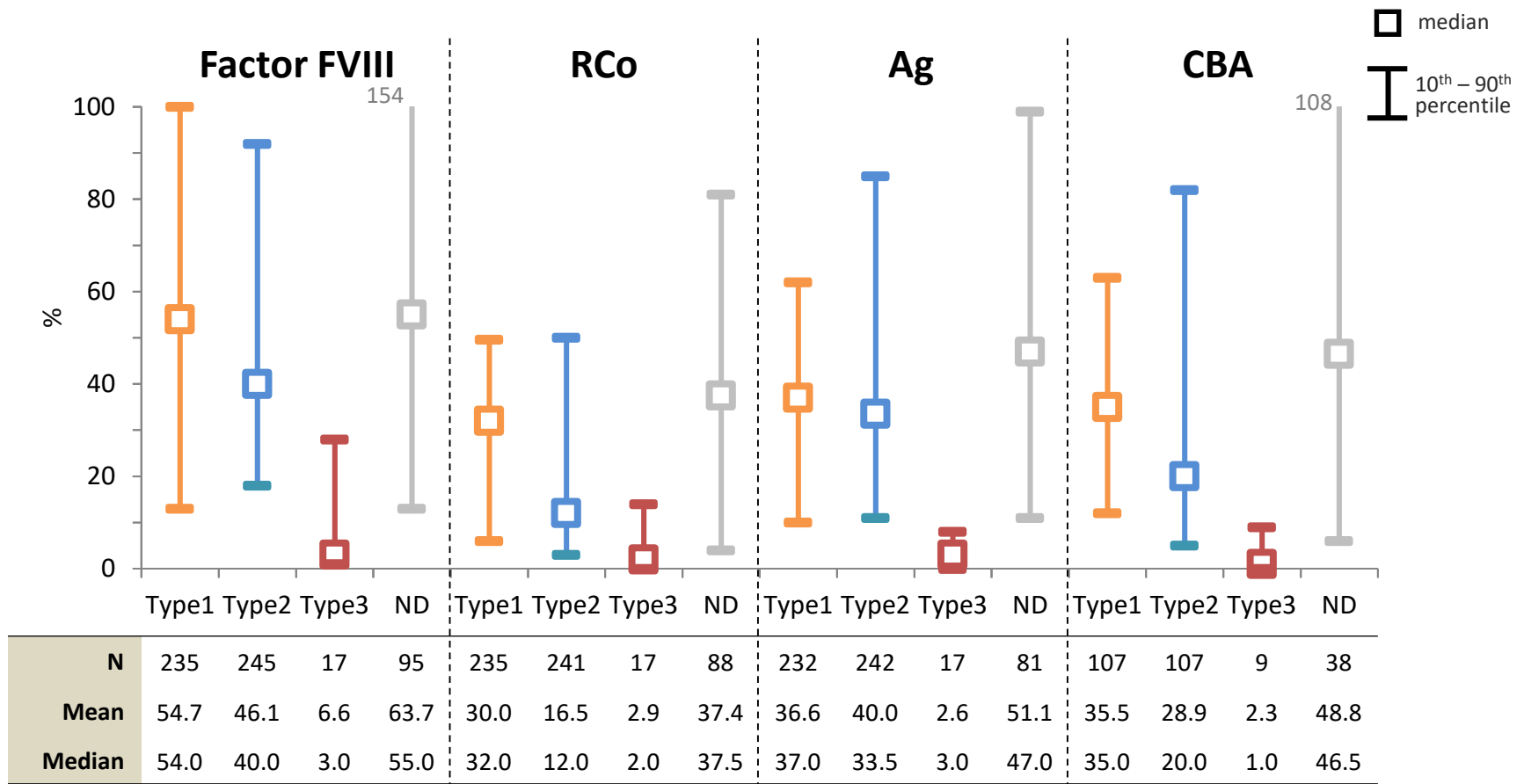
Factor FVIII*	RCo	Ag	CBA		RCo/Ag	CBA/Ag	FVIII/Ag
592	581	572	261	<b>N</b>	564	260	568
51.2	24.7	39.1	33.6	<b>Mean</b>	0.8	0.8	1.9
47 (1–232)	21 (0–135)	36 (0–261)	30 (0–127)	<b>Median (min – max)</b>	0.65 (0–38)	0.825 (0–3)	1.38 (0.08–106)

\* Factor FVIII was assessed by clotting assay in 549 patients, by chromogenic assay in 17 patients and assay is missing in 44 patients



# Factor levels according to type of VWD, part 1

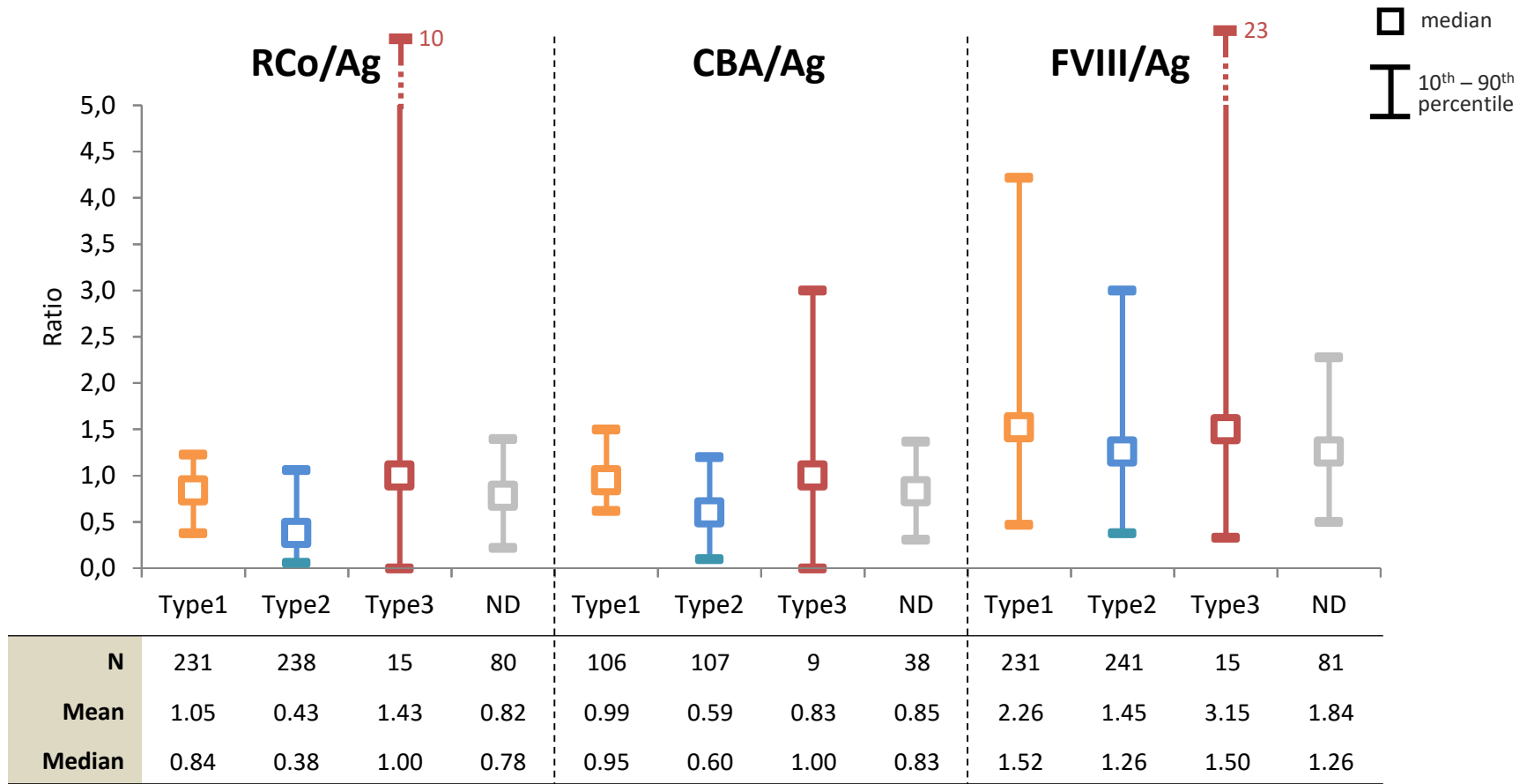
N=610



Type ND = not determined or unfilled

# Factor levels according to type of VWD, part 2

N=610

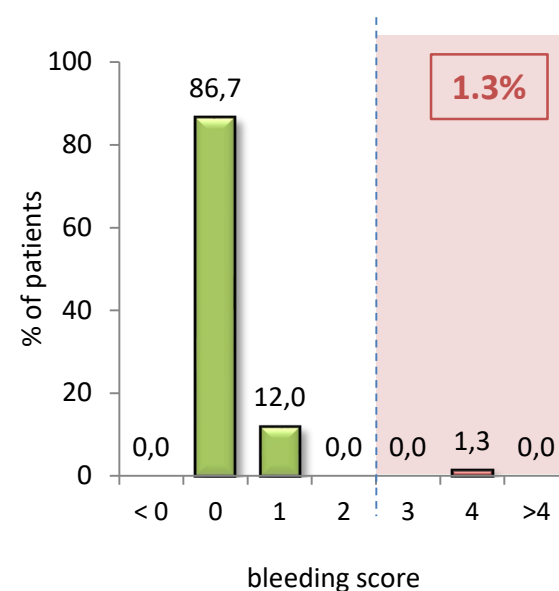
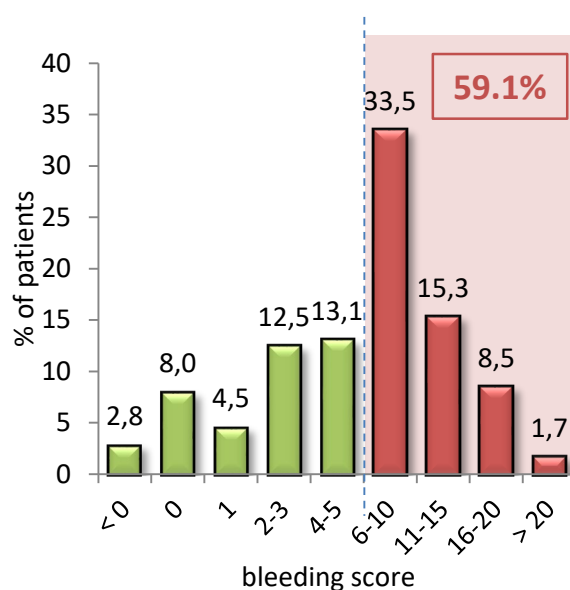
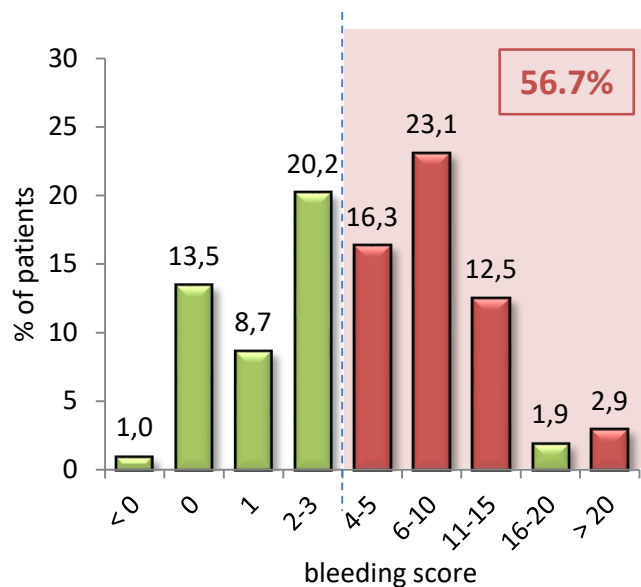


Type ND = not determined or unfilled

# Bleeding score<sup>1</sup> according to sex and age

N=355<sup>2</sup>

	Adult men	Adult women	Children
<b>N</b>	104	176	75
<b>Mean</b>	5.6	7.4	0.2
<b>Median (min - max)</b>	4.5 (-1 – 25)	6.5 (-3 – 24)	0 (0 – 4)



<sup>1</sup> Adult and Pediatric Vincenza VWD Bleeding Questionnaire and Scoring System

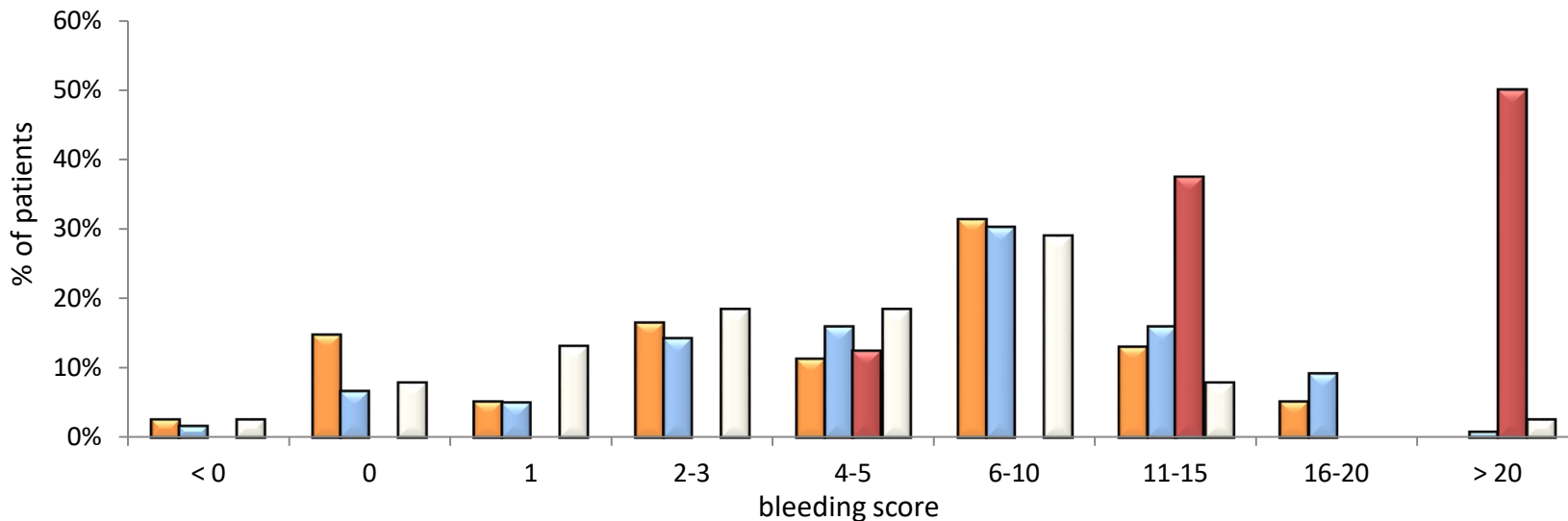
<sup>2</sup> Missing information on bleeding score in 255 patients.

# Bleeding score<sup>1</sup> in adults according to type of disease

N=280<sup>2</sup>

Bleeding score

	Type 1	Type 2	Type 3	Type ND	Total
N total	172	214	14	91	491
N valid	115	119	8	38	280
Mean	5.9	7.3	17.1	5.2	6.7
Median	5.0	6.0	17.5	4.0	6.0
min - max	-2-20	-1-22	5-25	-3-22	-3-25



<sup>1</sup> Adult and Pediatric Vincenza VWD Bleeding Questionnaire and Scoring System

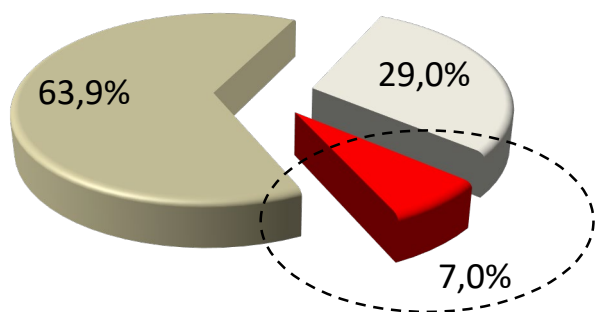
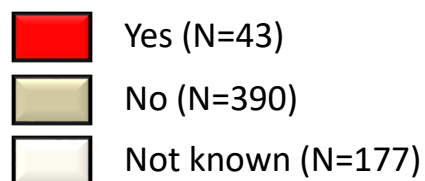
<sup>2</sup> Adult patients with non-missing information on bleeding score.

Type ND = not determined or unfilled

# Concomitant diseases

N=610

## Experienced hepatitis



*27x hepatitis A  
9x hepatitis B  
4x hepatitis A+B  
2x hepatitis C  
1x hepatitis A+C*

None of the patients is HIV positive.

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

	Type 1	Type 2	Type 3	Type ND	Total
<b>ABR</b>					
<b>N valid</b>	239	246	17	108	610
<b>Mean</b>	0.03	0.11	1.24	0.03	0.10
<b>Median</b>	0	0	0	0	0
<b>min – max</b>	0 - 1	0 - 7	0 - 6	0 - 1	0 - 7
<b>SUM</b>	7	28	21	3	59
<b>Location of bleeds</b>					
<b><math>N_p/N_B^2</math></b>					
<b>Joints</b>	1/1	0/-	0/-	0/-	1/1
<b>Epistaxes</b>	4/4	5/15	1/6	0/-	10/25
<b>Urogenital tract</b>	0/-	1/1	2/3	0/-	3/4
<b>GIT</b>	0/-	0/-	1/4	0/-	1/4
<b>Subcutaneous</b>	0/-	3/3	1/1	0/-	4/4
<b>Muscles</b>	0/-	0/-	4/6	1/1	5/7
<b>CNS</b>	0/-	1/1	0/-	1/1	2/2
<b>Other</b>	2/2	4/4	0/-	1/1	7/7
<b>SUM</b>	<b>7/7</b>	<b>14/24</b>	<b>9/20</b>	<b>3/3</b>	<b>33/54</b>

**N=610**

<sup>2</sup>  $N_p$  = number of patients with bleed;

Type ND = not determined or unfilled

$N_B$  = total number of bleeds treated with substitution of VWF

# Consumption of VWF concentrates in year 2018

N=610

	Number of patients treated with substitution of VWF total (type1/type2/type3/typeND)	Total annual consumption (IU)	Average annual consumption per treated patient (IU)
<b>Fanhdi</b>	<b>13 (7/5/0/1)</b>	<b>209 000</b>	<b>16 076.9</b>
<i>of them on prophylaxis*</i>	<i>1 (0/1/0/0)</i>	<i>80 000</i>	<i>80 000.0</i>
<b>Haemate P</b>	<b>61 (8/38/8/7)</b>	<b>1 471 600</b>	<b>24 124.6</b>
<i>of them on prophylaxis*</i>	<i>2 (0/0/2/0)</i>	<i>334 000</i>	<i>167 000.0</i>
<b>Wilate</b>	<b>18 (9/8/1/0)</b>	<b>170 100</b>	<b>9 450.0</b>
<i>of them on prophylaxis*</i>	<i>0 (0/0/0/0)</i>	<i>0</i>	<i>0.0</i>
<b>Willfact</b>	<b>1 (0/0/1/0)</b>	<b>60 000</b>	<b>60 000.0</b>
<i>of them on prophylaxis*</i>	<i>1 (0/0/1/0)</i>	<i>60 000</i>	<i>60 000.0</i>
<b>Total</b>	<b>94 (25/51/10/8)</b>	<b>1 910 700</b>	<b>18 753.0</b>
<i>of them on prophylaxis*</i>	<i>4 (0/1/3/0)</i>	<i>474 000</i>	<i>118 500.0</i>
Total - type 1	25	396 600	<u>12 793.8</u>
Total - type 2	51	703 250	<u>13 308.5</u>
Total - type 3	10	685 350	<u>68 535.0</u>
Total - type ND	8	125 500	<u>15 687.5</u>

\* permanent prophylaxis

Type ND = not determined or unfilled

# Consumption of other drugs in year 2018

N=610

	Number of patients treated with other drugs total (type1/type2/type3/typeND)	Total annual consumption	Average annual consumption per <u>treated</u> patient
<b>DDAVP (µg)</b>	<b>8 (6/2/0/0)</b>	<b>2 780</b>	<b>347.5</b>
<i>of them on prophylaxis*</i>	<i>0 (0/0/0/0)</i>	<i>0</i>	<i>0.0</i>
<b>NovoSeven (mg)</b>	<b>1 (1/0/0/0)</b>	<b>8</b>	<b>8.0</b>
<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=4

No	Centre	Age group	Type of VWD	Type of prophylaxis in 2018	Treatment in 2018	No of applications per week	Total consumption (IU)	ABR in 2018
1	Brno	Adult	3	<b>Permanent</b>	Haemate P	2	106 000	0
2	Brno	Adult	3	<b>Permanent</b>	Haemate P	2	228 000	5
3	Olomouc	Adult	3	<b>Permanent</b>	Willfact	1	60 000	0
4	Plzeň	Adult	2	<b>Permanent</b>	Fanhdi	NA	80 000	0