

The status of care for persons with von Willebrand disease registered within CNHP registry Annual Report 2016

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on behalf of

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

July 2017



TBA

Part B

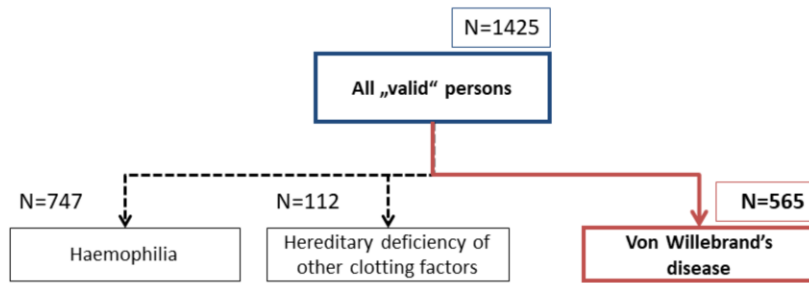
Persons with Von Willebrand disease



Czech National
Hemophilia
Program



Sample size



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

Number of patients in participating centres

N=565

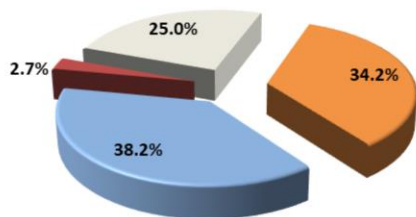
Paediatric centres	Valid patients	
	N	%
Brno – Dpt. of Pediatric Haematology, CUH Brno	27	4.8
Prague – Dpt. of Pediatric Haematology and Oncology, CUH Motol	23	4.1
Pilsen – Pediatric Dpt., UH Pilsen	22	3.9
Hradec Králové – Dpt. of Pediatric Medicine, UH HK	17	3.0
Ostrava – Dpt. of Pediatric Medicine, UH Ostrava	13	2.3
Ústí n.L. – Pediatric Dpt. – Haematology, Masaryk Hospital	10	1.8
Olomouc – Dpt. of Pediatric Medicine, UH Olomouc	1	0.2

Adult centres	Valid patients	
	N	%
Brno – Dpt. Of Clin Hematol, UH Brno	263	46.5
Pilsen – Dpt. of Biochemistry and Hematology, UH Pilsen – UKBH	75	13.3
Ostrava – Blood centre, UH Ostrava	65	11.5
Liberec – Dpt. Of Clin Hematol, Hospital Liberec	29	5.1
Olomouc – Haemato-Oncology Dpt., UH Olomouc	13	2.3
Ústí n.L. – Dpt. Of Clin Hematol, Masaryk Hospital	4	0.7
Hradec Králové – IV. Internal and Hematology Dpt., UH HK	2	0.4
České Budějovice – Dpt. Of Clin Hematol, Hospital CB	1	0.2

Centres participating in vWD survey within CNHP registry.

Type of Von Willebrand's disease

N=565



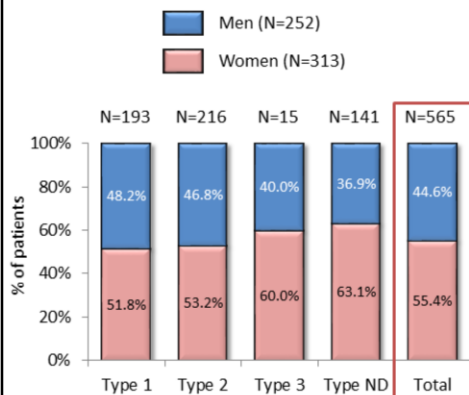
Type of disease	Patients	
	N	%
Type 1*	193	34.2
Type 2	216	38.2
Type 2A	97	17.2
Type 2B	15	2.7
Type 2M	31	5.5
Type 2N	18	3.2
Type 2 (not specified)	55	9.7
Type 3	15	2.7
Type not determined / unfilled	141	25.0
Total	565	100.0

* of them 1 patient with type 1/2N

Sex and current age of patients

N=565

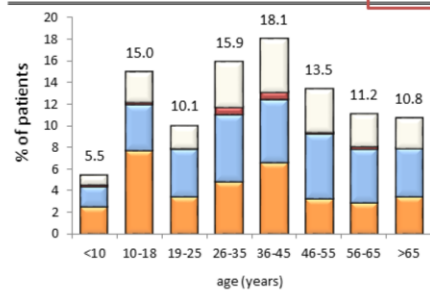
Sex



Type ND = not determined or unfilled

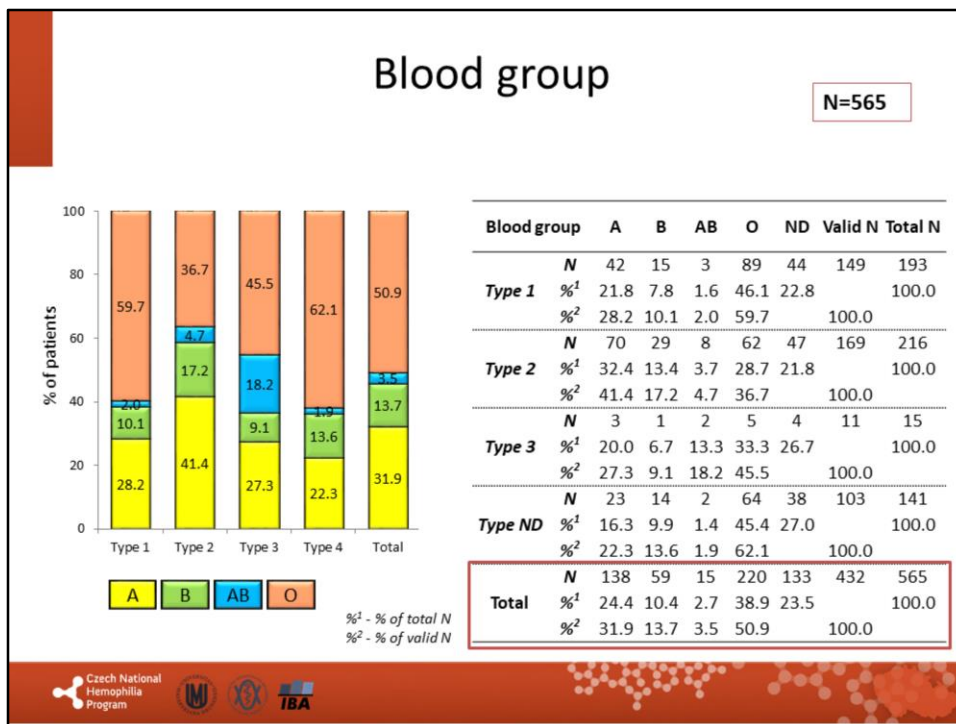
Current age*

	Type 1	Type 2	Type 3	Type ND	Total
N	193	216	15	141	565
Mean	34.5	40.0	34.7	40.5	38.1
Median	33	40	33	40	38
min - max	4 - 88	2 - 83	9 - 65	3 - 79	2 - 88

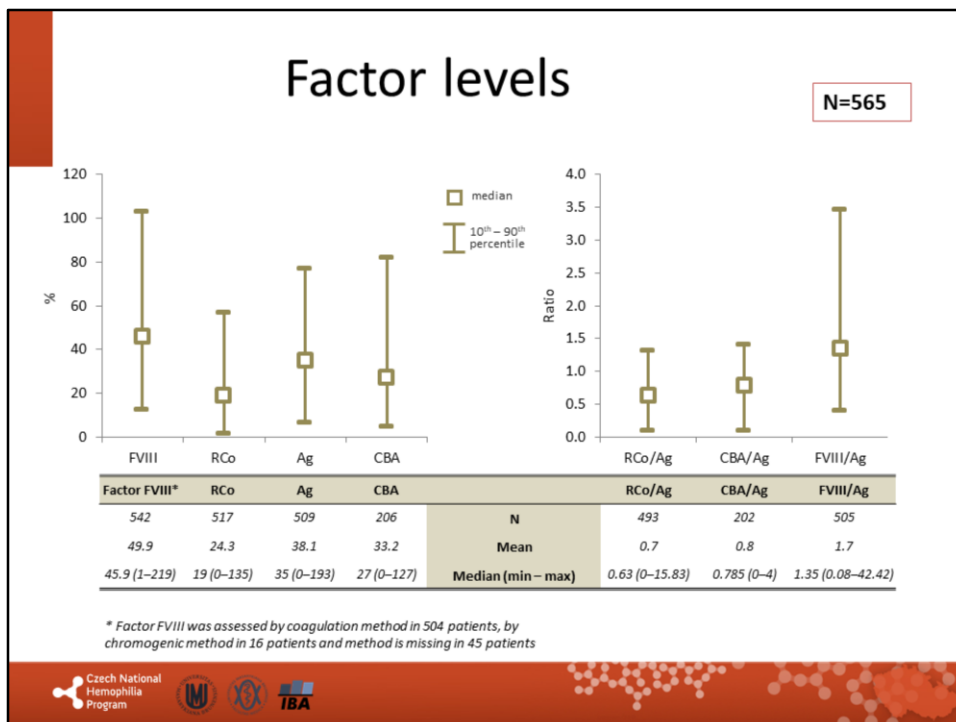


* age reached in year 2016

Median age of Czech persons with vWD is below 40 years.



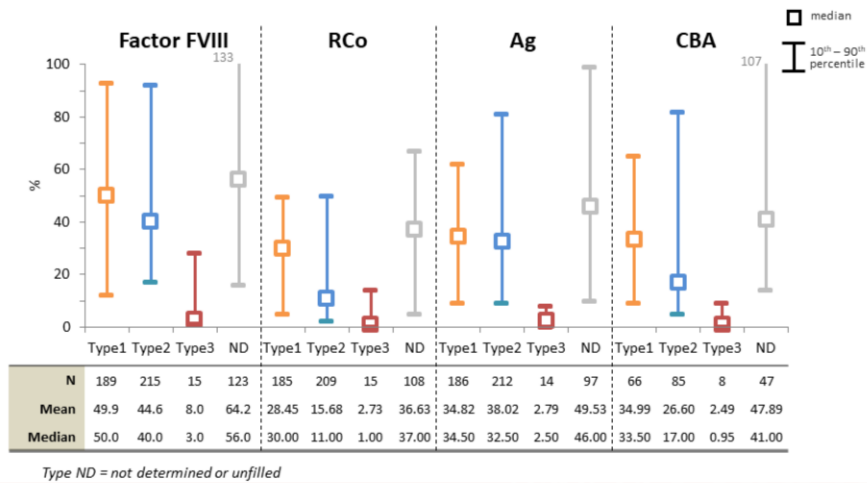
Well known predominance of BG O is confirmed within the registry.



This slide is giving just general overview as it is not related to different vWD subtypes.

Factor levels according to type of VWD, part 1

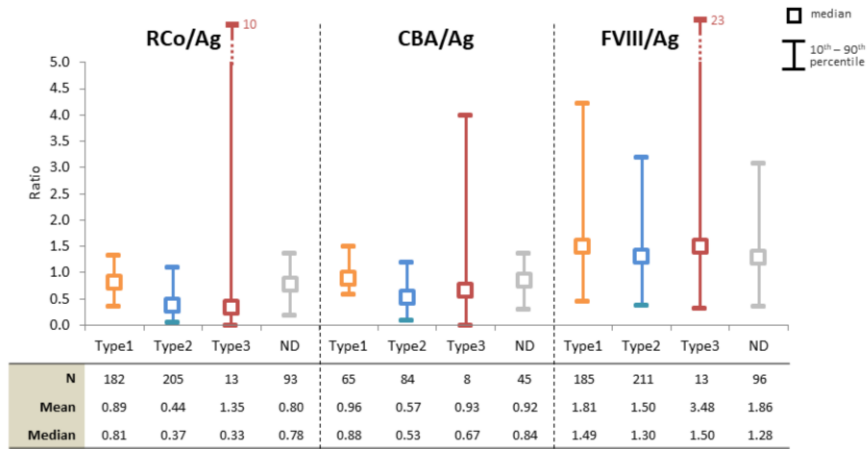
N=565



This and further slides show factor levels related to respective vWD subtypes. These data correlate well with published findings.

Factor levels according to type of VWD, part 2

N=565



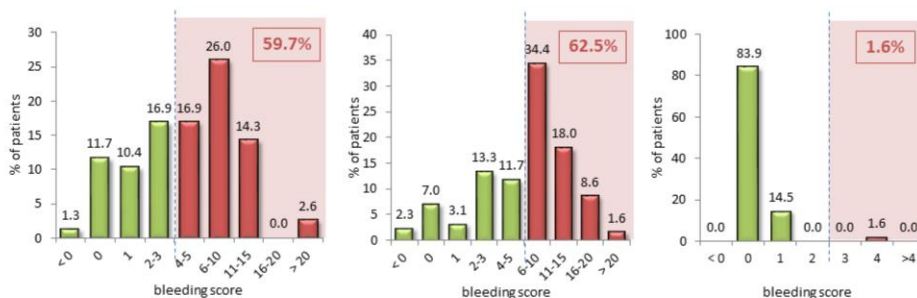
Type ND = not determined or unfilled



Bleeding score¹ according to sex and age

N=267²

	Adult men	Adult women	Children
N	77	128	62
Mean	5.5	7.7	0.2
Median (min - max)	5 (-1 - 25)	7 (-3 - 24)	0 (0 - 4)



¹ Adult and Pediatric Vincenza VWD Bleeding Questionnaire and Scoring System

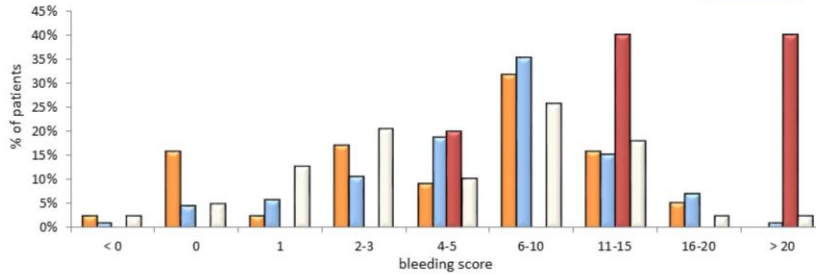
² Missing information on bleeding score in 298 patients.

Bleeding score¹ in adults according to type of disease

N=205²

Bleeding score

	Type 1	Type 2	Type 3	Type ND	Total
N total	136	181	12	120	449
N valid	76	85	5	39	205
Mean	6.1	7.4	16.2	5.9	6.9
Median	6.0	7.0	14.0	4.0	6.0
min - max	-1-18	-1-22	5-25	-3-22	-3-25

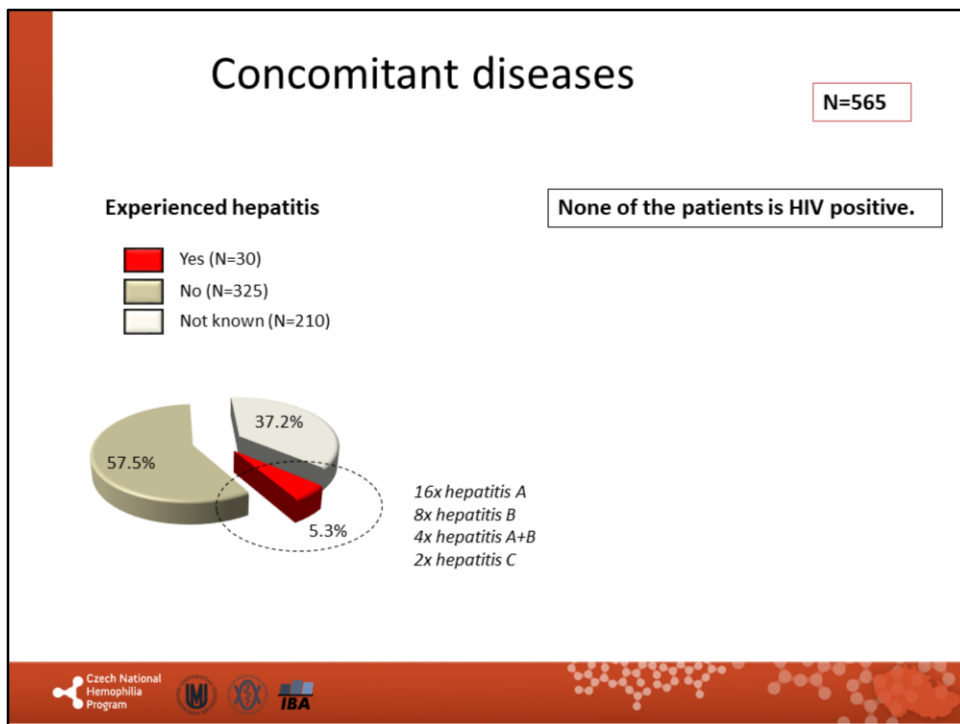


¹ Adult and Pediatric Vincenza VWD Bleeding Questionnaire and Scoring System

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

Type ND = not determined or unfilled

Type 3 has obviously the most heavy bleeding score.



Hepatitis and/or HIV prevalence in vWD patients within CNHP registry

Annual bleeding rate (ABR) and location of bleeds

N=472¹

	Type 1	Type 2	Type 3	Type ND	Total
ABR					
N valid	175	198	14	85	472
Mean	0.13	0.13	0.93	0.01	0.13
Median	0	0	0	0	0
min – max	0 - 12	0 - 5	0 - 6	0 - 1	0 - 12
SUM	22	25	13	1	61
Location of bleeds					
N_p/N_b^2					
Epistaxes	3/8	5/30	0/-	0/-	8/38
Other	1/1	5/6	0/-	0/-	6/7
Joints	1/12	1/1	2/4	0/-	4/17
Metrorrhagia	1/1	1/1	1/5	0/-	3/7
Muscles	0/-	0/-	2/2	1/1	3/3
Subcutaneous	0/-	0/-	2/2	0/-	2/2
Urogenital tract	0/-	1/3	0/-	0/-	1/3
SUM	6/22	13/41	7/13	1/1	27/77

¹ Missing information on ABR in 93 patients.

Type ND = not determined or unfilled

² N_p = number of patients with bleed; N_b = total number of bleeds treated with substitution of VWF



Annual bleeding rate and locations of bleeds in vWD patients within the registry. Please note, that not all of the bleeds needed treatment with vWF concentrate

Consumption of drugs in year 2016

N=565

	Number of treated patients total (type1/type2/type3/typeND)	Total annual consumption (IU)	Average annual consumption per treated patient (IU)
Fanhdi	18 (12/5/0/1)	165 500	9 194.4
of them on prophylaxis*	1 (0/1/0/0)	102 000	102 000.0
Haemate P	68 (24/30/7/7)	885 500	13 022.1
of them on prophylaxis*	2 (0/0/2/0)	262 000	131 000.0
Immunate	1 (1/0/0/0)	1 000	1 000.0
of them on prophylaxis*	0 (0/0/0/0)	0	0.0
Wilate	12 (7/4/1/0)	122 500	10 208.3
of them on prophylaxis*	0 (0/0/0/0)	0	0.0
Willfact	1 (0/0/1/0)	182 000	182 000.0
of them on prophylaxis*	1 (0/0/1/0)	182 000	182 000.0
Total	100 (44/39/9/8)	1 356 500	13 565.0
of them on prophylaxis*	4 (0/1/3/0)	546 000	136 500.0
Total - type 1	44	200 000	4 545.5
Total - type 2	39	613 000	15 717.9
Total - type 3	9	497 000	55 222.2
Total - type ND	8	46 500	5 812.5

* Permanent prophylaxis

Type ND = not determined or unfilled



Absolute numbers of respective concentrates consumption in this figure refer ONLY to the records within CNHP registry, which have been updated in 2016. The most important information on this slide is „Average annual consumption per treated person“. This aims to reflect nation-wide consumption of factor concentrate per treated patient. Shows well the highest consumption in type 3 vWD. Please note, that the numbers of treated patients are relatively small and do vary significantly between different concentrates. This is, indeed, a source of potential bias. To increase the data validity, we have to increase the number of treated patients recorded within CNHP registry.

On the other hand, for vWD treated patients (excluding type 3 patients) the annual consumption per treated patient should reflect the real situation (between 5 000 – 15 000 IU/year) relatively well. This slide also demonstrates significant difference in consumption of vWF concentrate between those, treated with prophylaxis and those, who do not need prophylaxis.

Patients on permanent prophylaxis in detail

N=4

No	Centre	Age group	Type of VWD	Type of prophylaxis in 2016	Treatment in 2016	No of applications per week	Total consumption (IU)	ABR in 2016
1	Brno	Child	3	Permanent	Willfact	2	182 000	0
2	Brno	Adult	3	Permanent	Haemate P	1.4	140 000	6
3	Brno	Adult	3	Permanent	Haemate P	2.33	122 000	0
4	Plzeň	Adult	2	Permanent	Fanhdi	3	102 000	0

This slide shows consumption of factor concentrate in vWD patients on prophylaxis (often permanent). Number of persons with vWD on prophylaxis is low in CZ.