

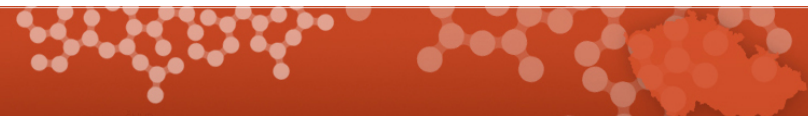
# The status of care for persons with von Willebrand disease registered within CNHP registry Annual Report 2019

Jan Blatný, Petra Ovesná

on behalf of

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

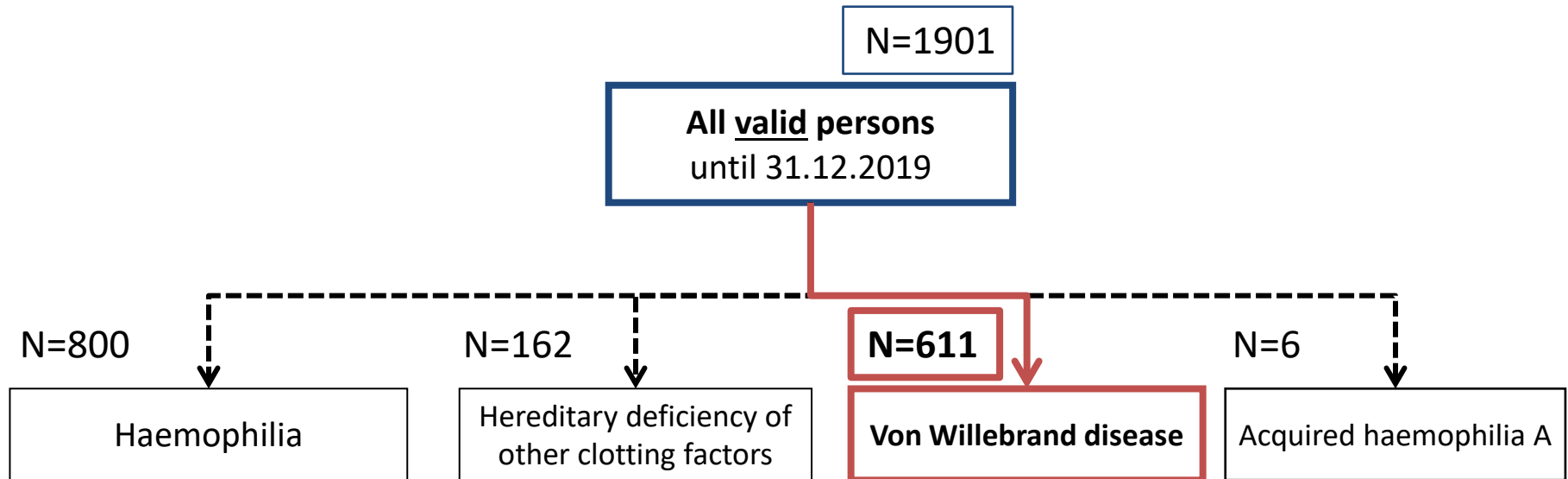
*Export date: May 11, 2020*



# Persons with Von Willebrand disease



# Sample size



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

# Number of patients in participating centres

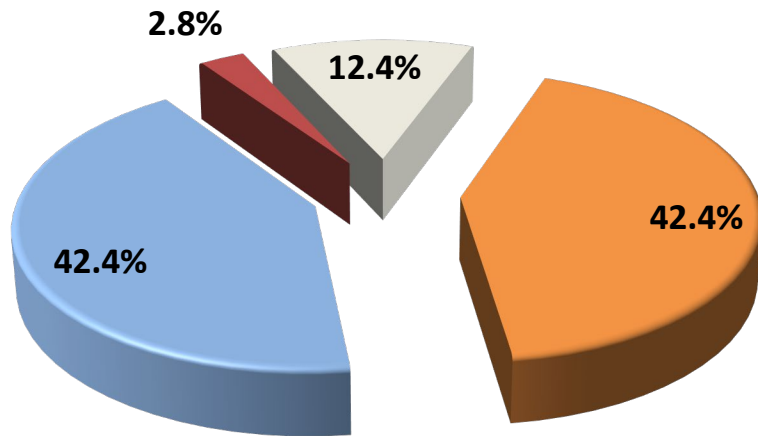
N=611





| Paediatric centres  | Valid patients |     |
|---|----------------|-----|
|   | N              | %   |
| <b>Prague</b> – Dpt. of Pediatric Haematology and Oncology, CUH Motol | 30             | 4.9 |
| <b>Pilsen</b> – Pediatric Dpt., UH Pilsen                             | 24             | 3.9 |
| <b>Ostrava</b> – Dpt. of Pediatric Medicine, UH Ostrava               | 21             | 3.4 |
| <b>Hradec Králové</b> – Dpt. of Pediatric Medicine, UH HK             | 20             | 3.3 |
| <b>Brno</b> – Dpt. of Pediatric Haematology, CUH Brno                 | 17             | 2.8 |
| <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                           | 272            | 44.5 |
| <b>Pilsen</b> – Dpt. of Biochemistry and Hematology, UH Pilsen – UKBH | 77             | 12.6 |
| <b>Ostrava</b> – Blood centre, UH Ostrava                             | 68             | 11.1 |
| <b>Liberec</b> – Dpt. Of Clin Hematol, Hospital Liberec               | 25             | 4.1  |
| <b>Olomouc</b> – Haemato-Oncology Dpt., UH Olomouc                    | 23             | 3.8  |
| <b>Ústí n.L.</b> – Dpt. Of Clin Hematol, Masaryk Hospital             | 16             | 2.6  |
| <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's disease

N=611



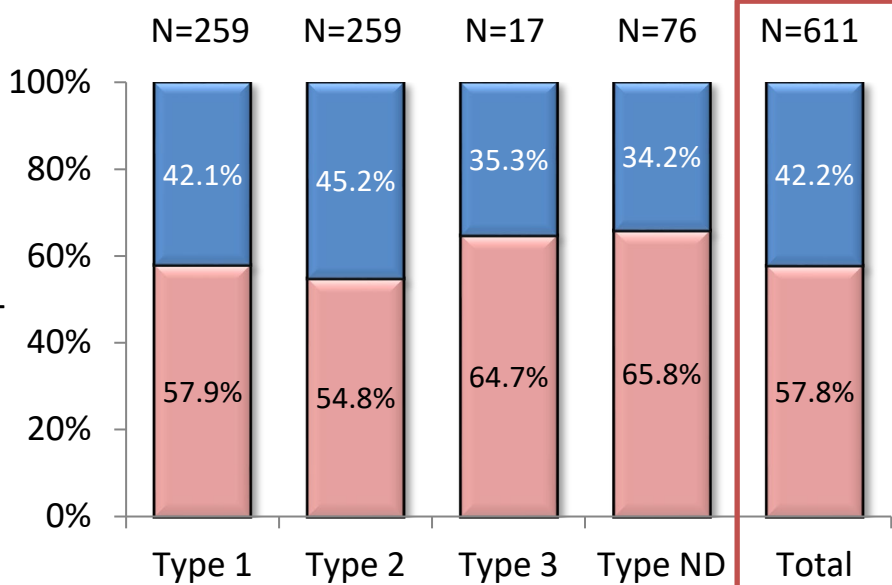
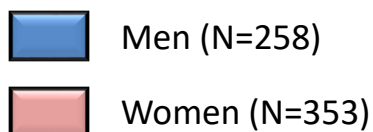
|   |                                | Patients |       |
|---|--------------------------------|----------|-------|
| Type of disease   |                                | N        | %     |
|    | Type 1*                        | 259      | 42.4  |
|    | Type 2                         | 259      | 42.4  |
|   | Type 2A                        | 110      | 18.0  |
|   | Type 2B                        | 20       | 3.3   |
|   | Type 2M                        | 41       | 6.7   |
|   | Type 2N                        | 22       | 3.6   |
|   | Type 2 (not specified)         | 66       | 10.8  |
|  | Type 3                         | 17       | 2.8   |
|  | Type not determined / unfilled | 76       | 12.4  |
| Total   |                                | 611      | 100.0 |

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

# Sex and current age of patients

**N=611**

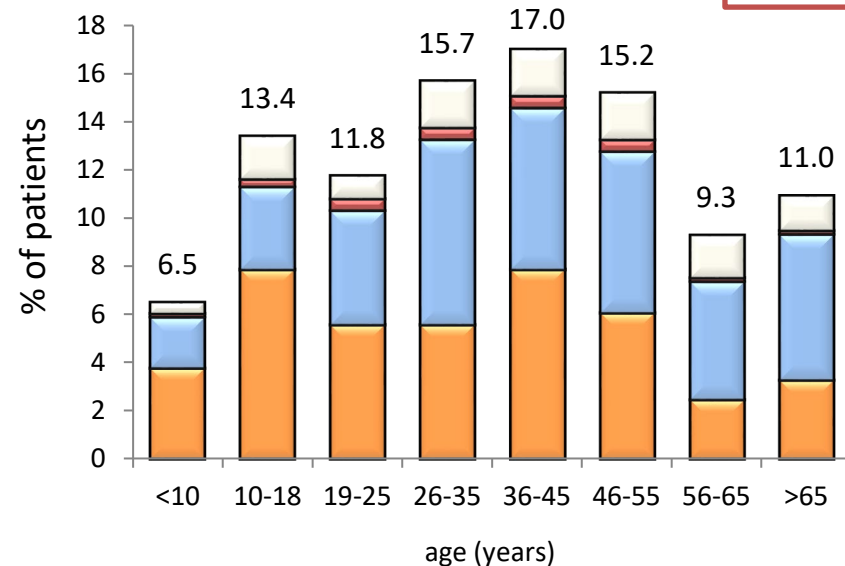
## Sex



Type ND = not determined or unfilled

## Current age\*

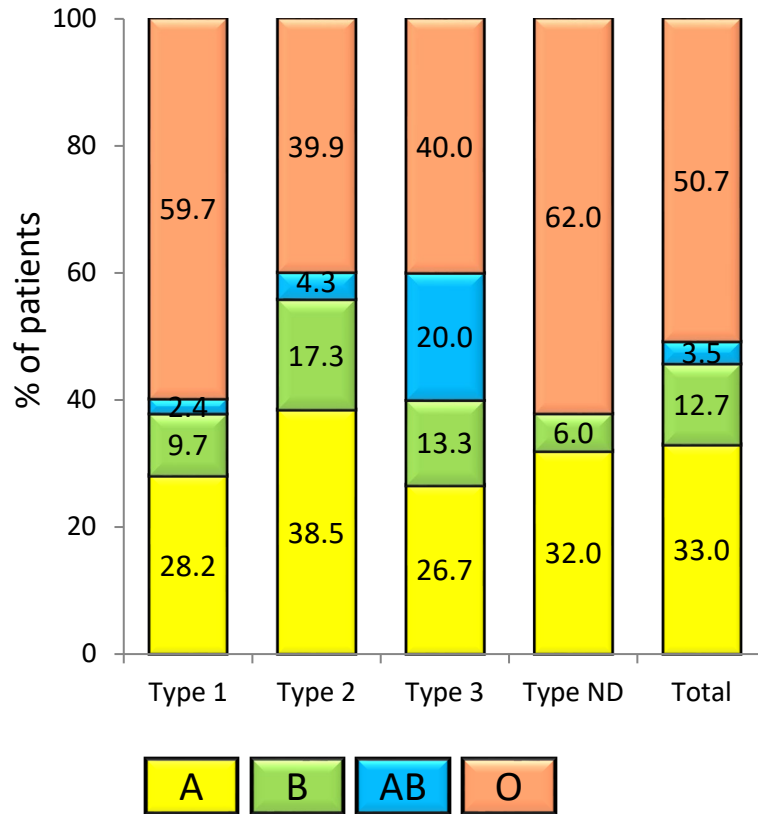
|                  | Type 1 | Type 2 | Type 3 | Type ND | Total         |
|------------------|--------|--------|--------|---------|---------------|
| <b>N</b>         | 259    | 259    | 17     | 76      | <b>611</b>    |
| <b>Mean</b>      | 34.1   | 41.6   | 34.5   | 40.9    | <b>38.1</b>   |
| <b>Median</b>    | 33     | 41     | 33     | 40      | <b>37</b>     |
| <b>min - max</b> | 2 - 91 | 2 - 86 | 3 - 68 | 5 - 82  | <b>2 - 91</b> |



\* age reached in year 2019

# Blood group

N=611

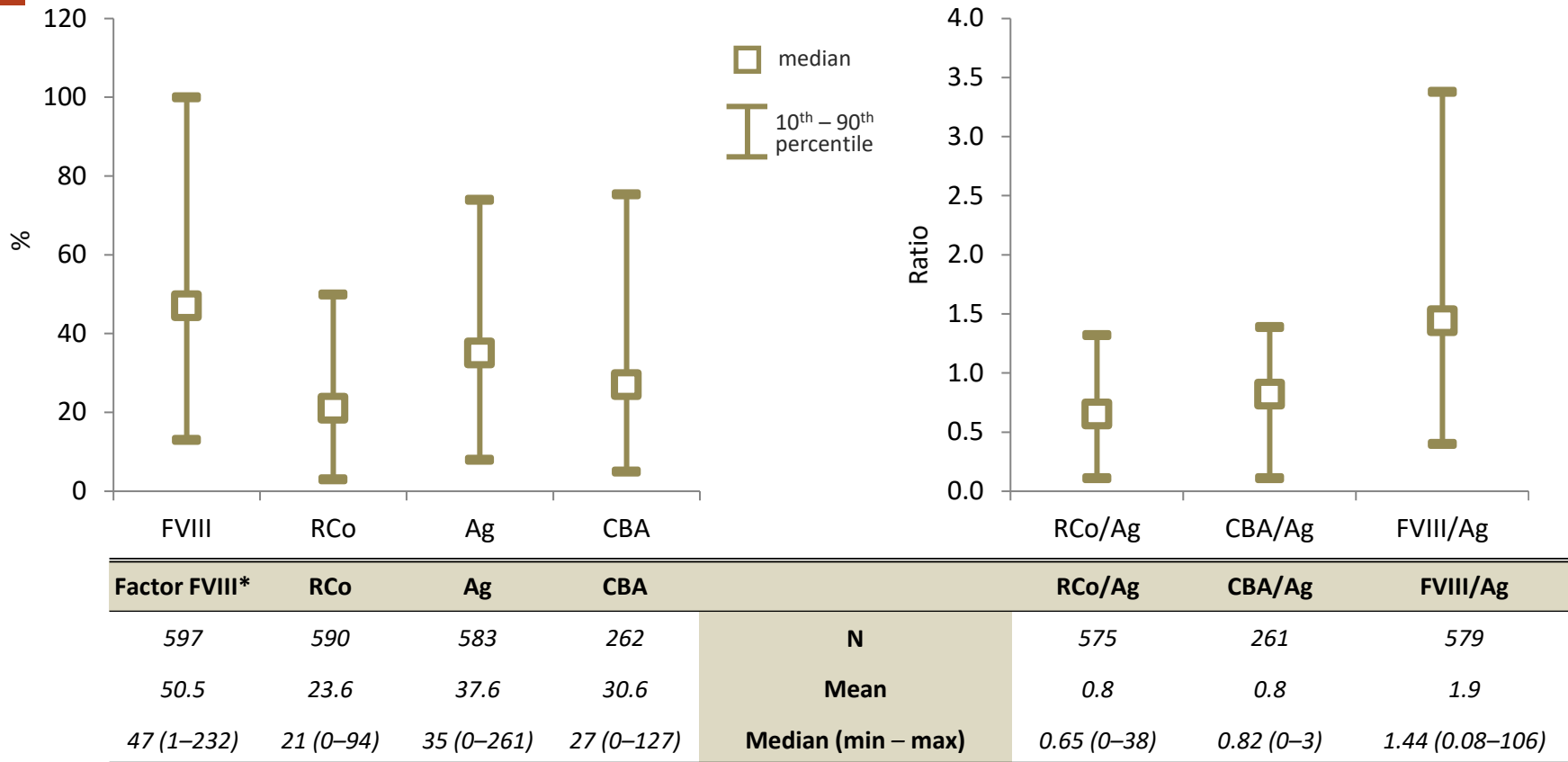


%<sup>1</sup> - % of total N  
 %<sup>2</sup> - % of valid N

| Blood group    | A              | B    | AB   | O    | ND   | Valid N | Total N |
|----------------|----------------|------|------|------|------|---------|---------|
| <b>Type 1</b>  | <i>N</i>       | 58   | 20   | 5    | 123  | 53      | 206     |
|                | % <sup>1</sup> | 22.4 | 7.7  | 1.9  | 47.5 | 20.5    | 100.0   |
|                | % <sup>2</sup> | 28.2 | 9.7  | 2.4  | 59.7 |         | 100.0   |
| <b>Type 2</b>  | <i>N</i>       | 80   | 36   | 9    | 83   | 51      | 208     |
|                | % <sup>1</sup> | 30.9 | 13.9 | 3.5  | 32.0 | 19.7    | 100.0   |
|                | % <sup>2</sup> | 38.5 | 17.3 | 4.3  | 39.9 |         | 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>       | 16   | 3    | 0    | 31   | 26      | 50      |
|                | % <sup>1</sup> | 21.1 | 3.9  | 0.0  | 40.8 | 34.2    | 100.0   |
|                | % <sup>2</sup> | 32.0 | 6.0  | 0.0  | 62.0 |         | 100.0   |
| <b>Total</b>   | <i>N</i>       | 158  | 61   | 17   | 243  | 132     | 479     |
|                | % <sup>1</sup> | 25.9 | 10.0 | 2.8  | 39.8 | 21.6    | 100.0   |
|                | % <sup>2</sup> | 33.0 | 12.7 | 3.5  | 50.7 |         | 100.0   |

# Factor levels

N=611

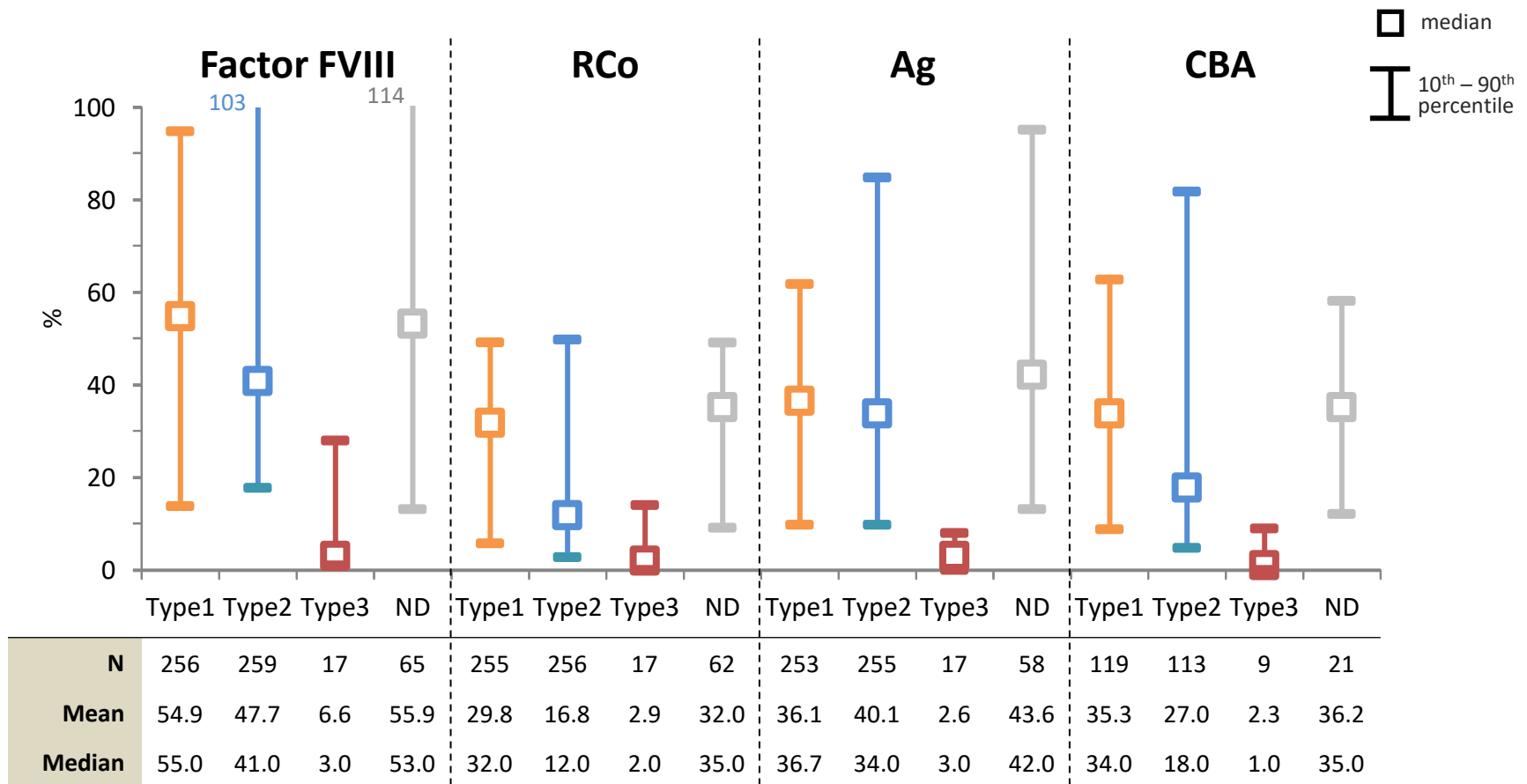


\* Factor FVIII was assessed by coagulation method in 549 patients, by chromogenic method in 17 patients and method is missing in 44 patients



# Factor levels according to type of VWD, part 1

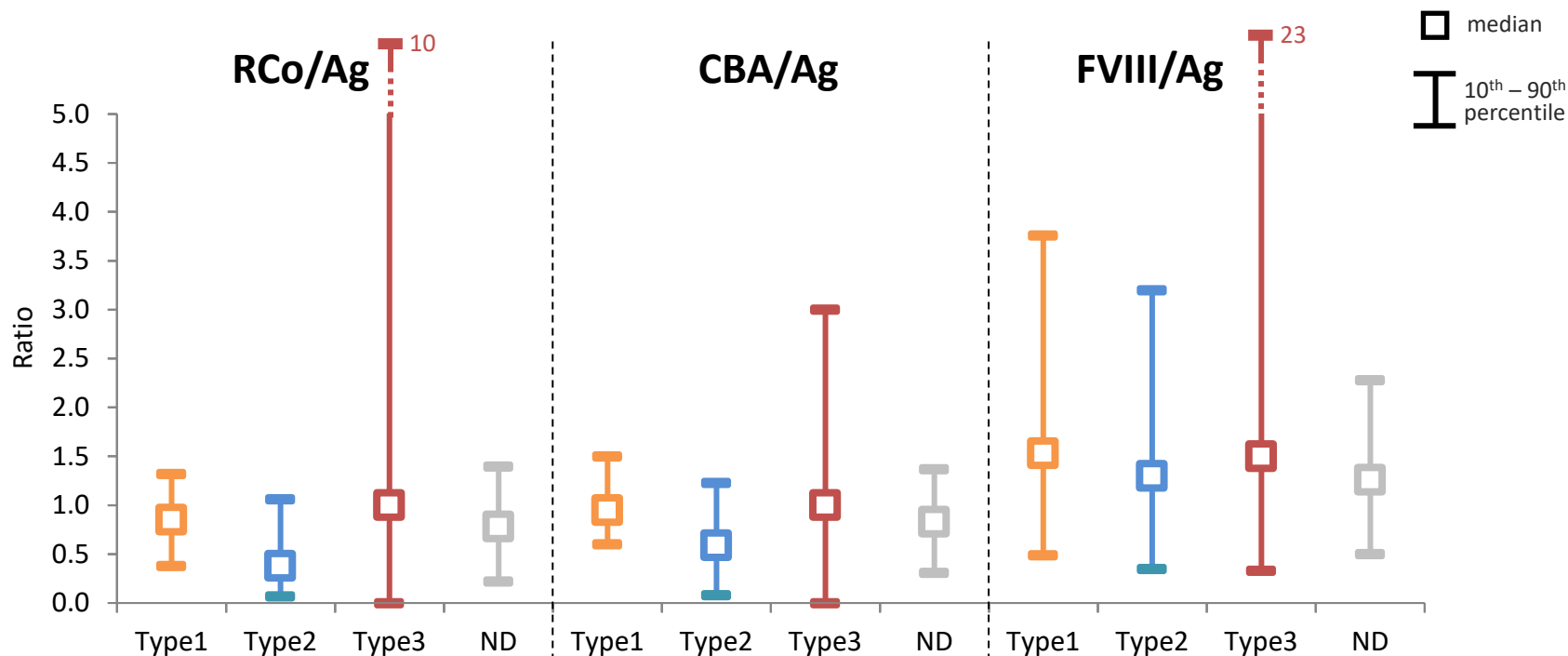
N=611



Type ND = not determined or unfilled

# Factor levels according to type of VWD, part 2

N=611



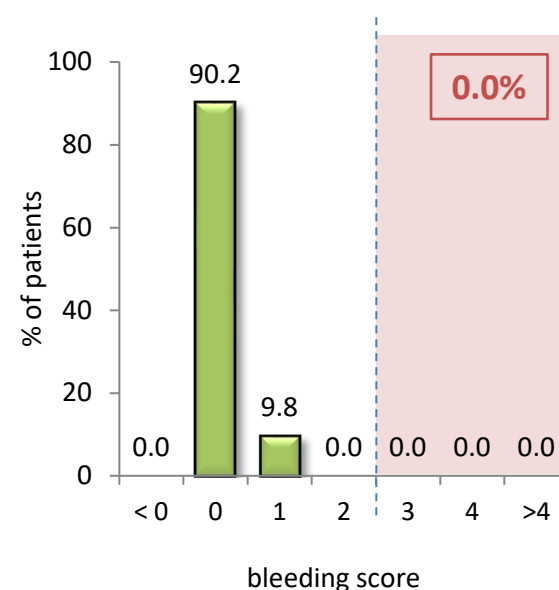
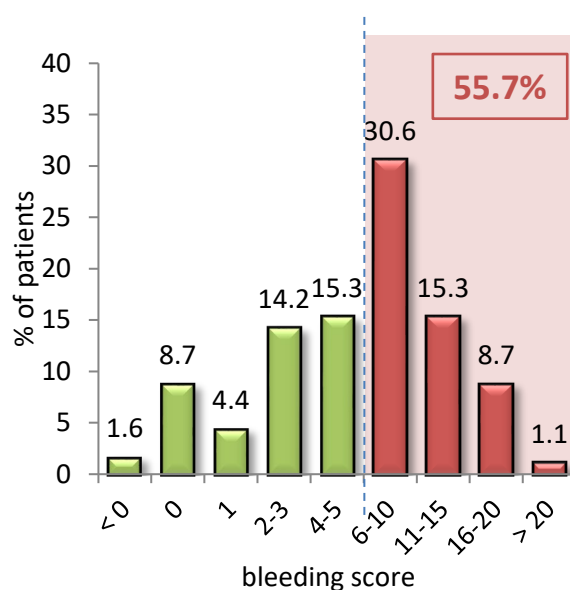
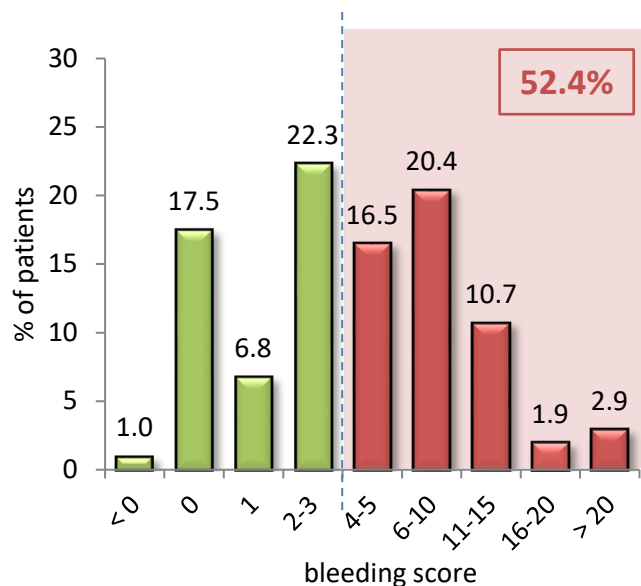
|               | Type1 | Type2 | Type3 | ND   | Type1 | Type2 | Type3 | ND   | Type1 | Type2 | Type3 | ND   |
|---------------|-------|-------|-------|------|-------|-------|-------|------|-------|-------|-------|------|
| <b>N</b>      | 251   | 252   | 15    | 57   | 118   | 113   | 9     | 21   | 252   | 254   | 15    | 58   |
| <b>Mean</b>   | 1.04  | 0.43  | 1.43  | 0.85 | 0.98  | 0.58  | 0.83  | 0.86 | 2.23  | 1.52  | 3.15  | 1.35 |
| <b>Median</b> | 0.85  | 0.38  | 1.00  | 0.77 | 0.95  | 0.59  | 1.00  | 0.79 | 1.53  | 1.30  | 1.50  | 1.27 |

Type ND = not determined or unfilled

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

N=368<sup>2</sup>

|                               | Adult men   | Adult women | Children  |
|-------------------------------|-------------|-------------|-----------|
| <b>N</b>                      | 103         | 183         | 82        |
| <b>Mean</b>                   | 5.2         | 7.2         | 0.1       |
| <b>Median<br/>(min - max)</b> | 4 (-1 - 25) | 6 (-2 - 24) | 0 (0 - 1) |



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

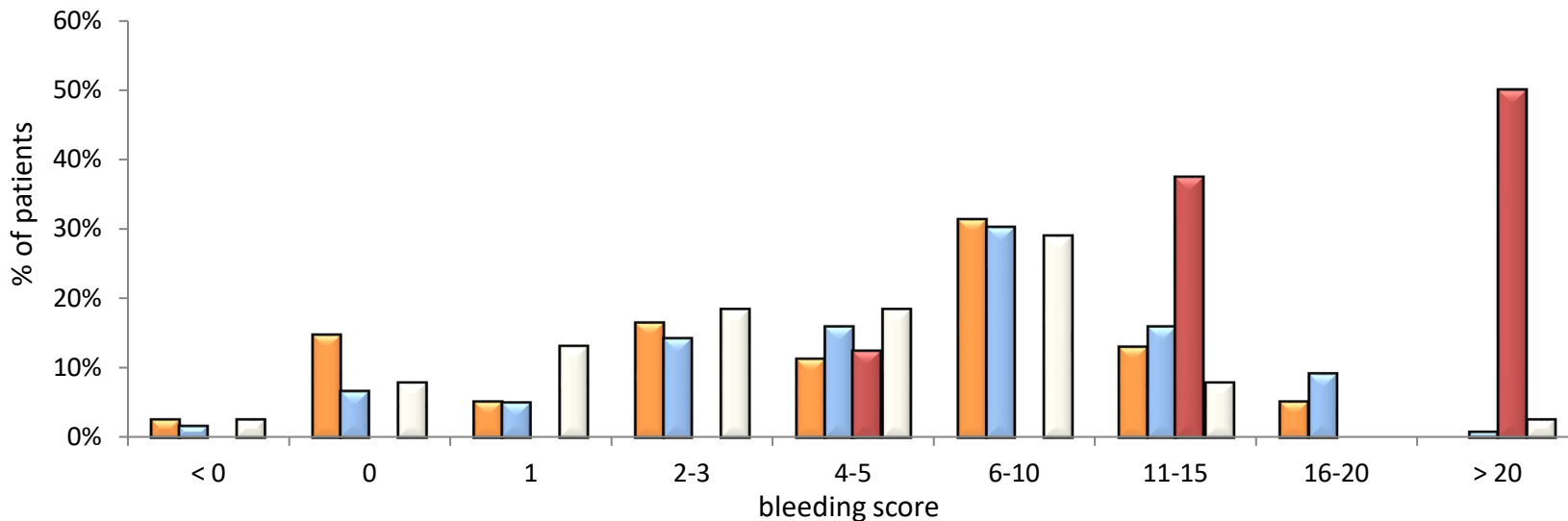
<sup>2</sup> Missing information on bleeding score in 243 patients.

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

N=286<sup>2</sup>

Bleeding score

|                  | Type 1     | Type 2     | Type 3      | Type ND    | Total        |
|------------------|------------|------------|-------------|------------|--------------|
| <b>N total</b>   | 188        | 225        | 14          | 62         | <b>489</b>   |
| <b>N valid</b>   | 129        | 129        | 8           | 20         | <b>286</b>   |
| <b>Mean</b>      | <b>5.6</b> | <b>6.8</b> | <b>17.1</b> | <b>5.8</b> | <b>6.5</b>   |
| <b>Median</b>    | <b>5.0</b> | <b>6.0</b> | <b>17.5</b> | <b>5.0</b> | <b>5.0</b>   |
| <b>min - max</b> | -2-20      | -1-22      | 5-25        | 0-14       | <b>-2-25</b> |



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

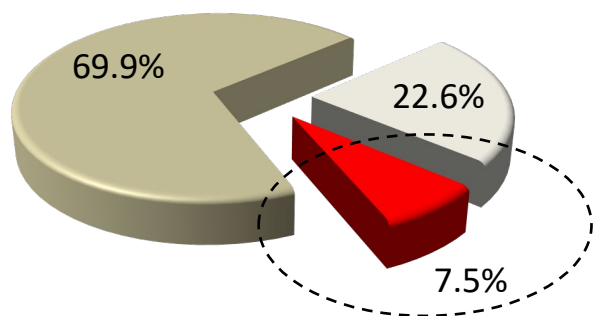
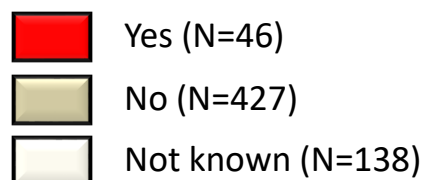
<sup>2</sup> Adult patients with non-missing information on bleeding score.

Type ND = not determined or unfilled

# Concomitant diseases

N=611

## Experienced hepatitis



*28x hepatitis A  
9x hepatitis B  
5x hepatitis A+B  
2x hepatitis C  
1x hepatitis A+C  
1x hepatitis A+B+C*

None of the patients is HIV positive.

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

**N=611**

|                               | Type 1      | Type 2       | Type 3       | Type ND    | Total        |
|-------------------------------|-------------|--------------|--------------|------------|--------------|
| <b>ABR</b>                    |             |              |              |            |              |
| <b>N valid</b>                | 259         | 259          | 17           | 76         | 611          |
| <b>Mean</b>                   | 0.06        | 0.22         | 2.12         | 0.04       | 0.18         |
| <b>Median</b>                 | 0           | 0            | 0            | 0          | 0            |
| <b>min – max</b>              | 0 - 4       | 0 - 10       | 0 - 14       | 0 - 3      | 0 - 14       |
| <b>SUM</b>                    | 15          | 57           | 36           | 3          | 111          |
| <hr/>                         |             |              |              |            |              |
| <b>Location of bleeds</b>     |             |              |              |            |              |
| <b><math>N_p/N_B^2</math></b> |             |              |              |            |              |
| <b>Joints</b>                 | 0/-         | 3/3          | 4/8          | 0/-        | 7/11         |
| <b>Epistaxes</b>              | 4/9         | 12/20        | 2/10         | 1/1        | 19/40        |
| <b>Urogenital tract</b>       | 0/-         | 2/2          | 1/2          | 0/-        | 3/4          |
| <b>GIT</b>                    | 0/-         | 2/2          | 1/1          | 0/-        | 3/3          |
| <b>Subcutaneous</b>           | 0/-         | 1/1          | 1/2          | 0/-        | 2/3          |
| <b>Muscles</b>                | 0/-         | 2/2          | 2/5          | 0/-        | 4/7          |
| <b>CNS</b>                    | 0/-         | 0/-          | 0/-          | 0/-        | 0/-          |
| <b>Metrorrhagia</b>           | 0/-         | 0/-          | 1/4          | 0/-        | 1/4          |
| <b>Other</b>                  | 2/2         | 6/6          | 3/4          | 1/2        | 12/14        |
| <b>SUM</b>                    | <b>6/11</b> | <b>28/36</b> | <b>15/36</b> | <b>2/3</b> | <b>51/86</b> |

<sup>2</sup>  $N_p$  = 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 2019

N=611

|                                | Number of patients treated with substitution of VWF<br>total (type1/type2/type3/typeND) | Total annual consumption (IU) | Average annual consumption per treated patient (IU) |
|--------------------------------|---|-------------------------------|---|
| <b>Fanhdi</b>                  | <b>8 (3/5/0/0)</b>  | <b>68 000</b>                 | <b>8 500.0</b>                                      |
| <i>of them on prophylaxis*</i> | <i>1 (0/1/0/0)</i>  | <i>39 000</i>                 | <i>39 000.0</i>                                     |
| <b>Haemate P</b>               | <b>72 (16/42/8/6)</b>   | <b>2 023 000</b>              | <b>28 097.2</b>                                     |
| <i>of them on prophylaxis*</i> | <i>3 (0/1/2/0)</i>  | <i>541 500</i>                | <i>180 500.0</i>                                    |
| <b>Immunate</b>                | <b>2 (2/0/0/0)</b>  | <b>2 500</b>                  | <b>1 250.0</b>                                      |
| <i>of them on prophylaxis*</i> | <i>0 (0/0/0/0)</i>  | <i>0</i>                      | <i>0.0</i>  |
| <b>Wilate</b>                  | <b>21 (9/9/3/0)</b>   | <b>220 000</b>                | <b>10 476.2</b>                                     |
| <i>of them on prophylaxis*</i> | <i>1 (0/0/1/0)</i>  | <i>91 000</i>                 | <i>91 000.0</i>                                     |
| <b>Willfact</b>                | <b>1 (0/0/1/0)</b>  | <b>2 000</b>                  | <b>2 000.0</b>                                      |
| <i>of them on prophylaxis*</i> | <i>1 (0/0/1/0)</i>  | <i>2 000</i>                  | <i>2 000.0</i>                                      |
| <b>Total</b>                   | <b>104 (30/56/12/6)</b>   | <b>2 313 000</b>              | <b>21 459.3</b>                                     |
| <i>of them on prophylaxis*</i> | <i>6 (0/2/4/0)</i>  | <i>673 500</i>                | <i>112 250.0</i>                                    |
| Total - type 1                 | 30  | 251 502                       | <u>8 193.6</u>                                      |
| Total - type 2                 | 56  | 1 087 999                     | <u>18 476.3</u>                                     |
| Total - type 3                 | 12  | 877 000                       | <u>73 083.3</u>                                     |
| Total - type ND                | 6   | 96 500                        | <u>16 083.3</u>                                     |

\* permanent prophylaxis

Type ND = not determined or unfilled

# Consumption of other drugs in year 2019

N=611

|                                | 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>4 (1/3/0/0)</b>   | <b>2 102</b>             | <b>525.4</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 2019 | Treatment in 2019        | No of applications per week | Total consumption (IU) | ABR in 2019 |
|----|---------|-----------|-------------|-----------------------------|--------------------------|-----------------------------|------------------------|-------------|
| 1  | Brno    | Adult     | 3           | <b>Permanent</b>            | Haemate P                | 2                           | 235 000                | 3           |
| 2  | Brno    | Adult     | 3           | <b>Permanent</b>            | Haemate P                | 2                           | 294 000                | 4           |
| 3  | Olomouc | Adult     | 3           | <b>Permanent</b>            | Willfact, Wilate         | N/A                         | 93 000                 | 0           |
| 4  | Plzeň   | Adult     | 2           | <b>Permanent</b>            | Fanhdi, Haemate P, DDAVP | 1.5                         | 51 500 (w/o DDAVP)     | 0           |