# Léčba dětí s hemofilií v České republice
# -
# Aktuální data z registru ČNHP

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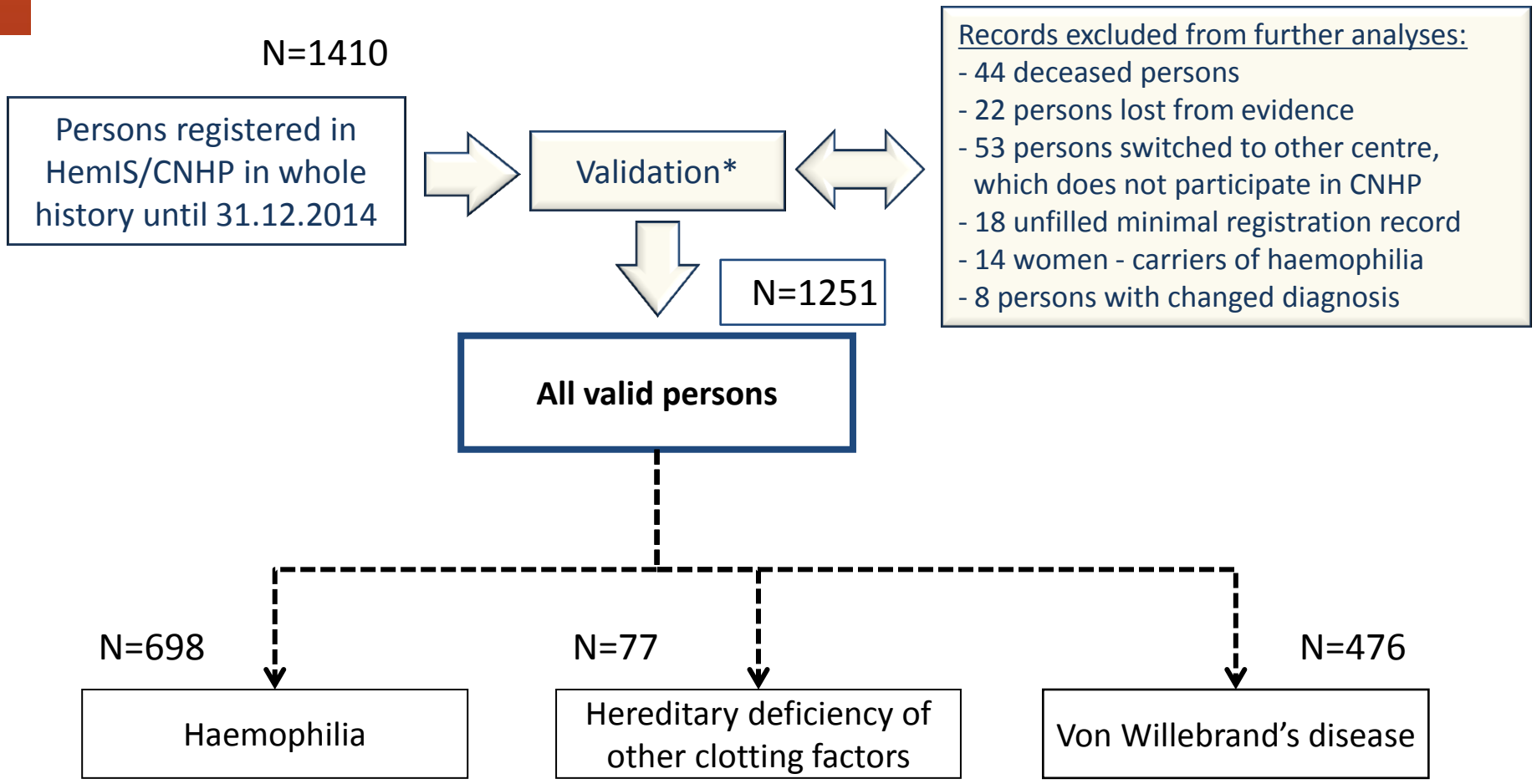
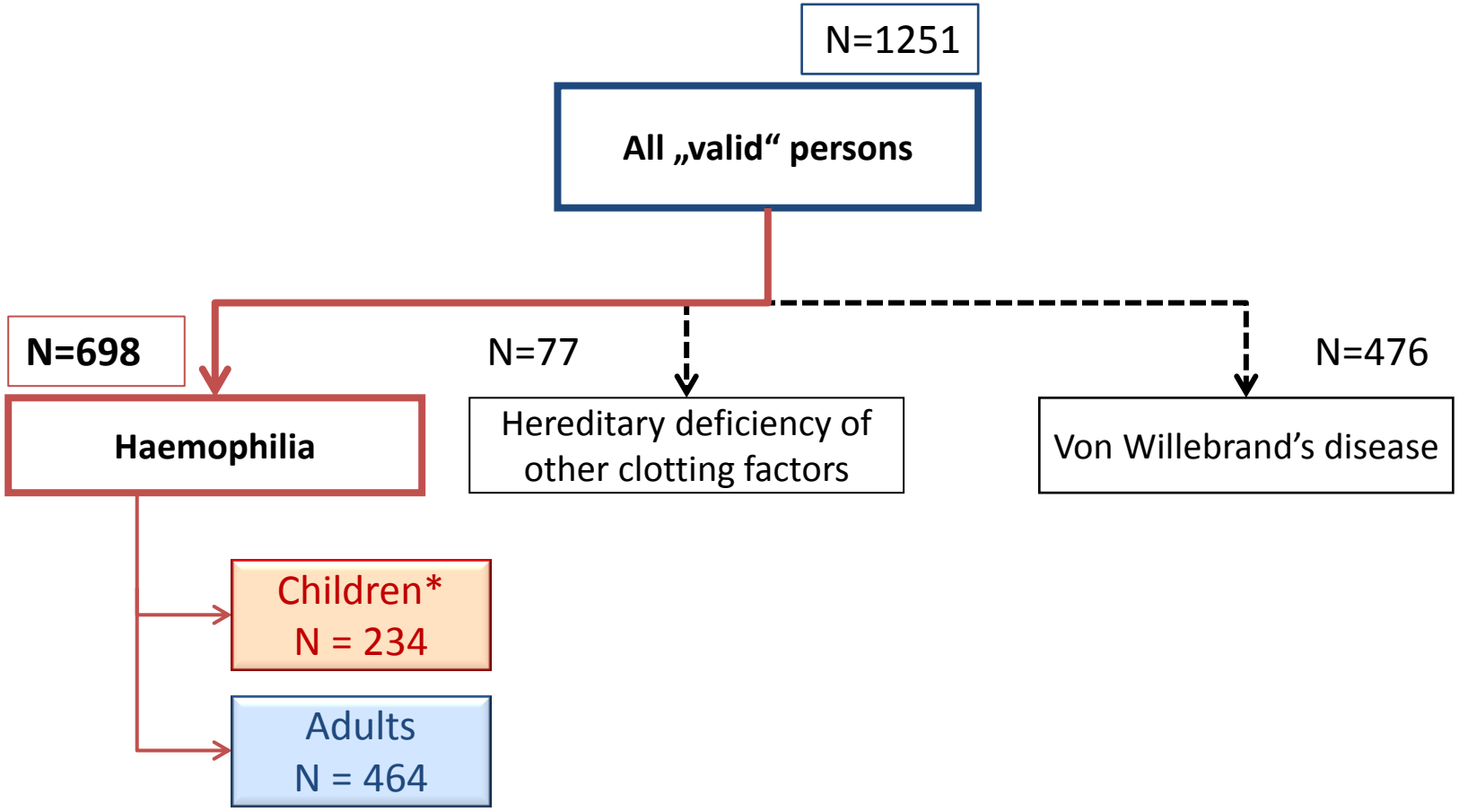
jménem a pro
Centra přispívající do databáze registru ČNHP CNHP
(Český národní hemofilický program)

*Květen 2015*

# Sample size, valid records



# Sample size




* *Persons under 19 years old in 2014*

# Participating centres in CNHP

| Paediatric centres | Valid persons N | % |
|---|---|---|
| FN Motol – Dpt. of Pediatric Haematology and Oncology | 86 | 12.3 |
| FN Brno – DN – Dpt. of Pediatric Haematology | 46 | 6.6 |
| FNHK – Dpt. of Pediatric Medicine | 27 | 3.9 |
| FN Ostrava – Dpt. of Pediatric Medicine | 27 | 3.9 |
| UnL – Pediatric Dpt. – Haematology | 27 | 3.9 |
| CB – Pediatric Dpt. | 13 | 1.9 |
| FN Plzen – Pediatric Dpt. | 13 | 1.9 |
| FN Olomouc – Dpt. of Pediatric Medicine | 12 | 1.7 |

| Adult centres | Valid persons N | % |
|---|---|---|
| FN Brno – OKH | 138 | 19.8 |
| FN Ostrava – Blood centre | 67 | 9.6 |
| FN Olomouc – Haemato-Oncology Dpt. | 61 | 8.7 |
| FN Plzen – UKBH | 48 | 6.9 |
| FN a LF HK – IV. IHK | 39 | 5.6 |
| KN Liberec – OKH | 36 | 5.2 |
| CB – OKH | 29 | 4.2 |
| UnL – OKH | 20 | 2.9 |
| Plzen - hemacentrum | 9 | 1.3 |

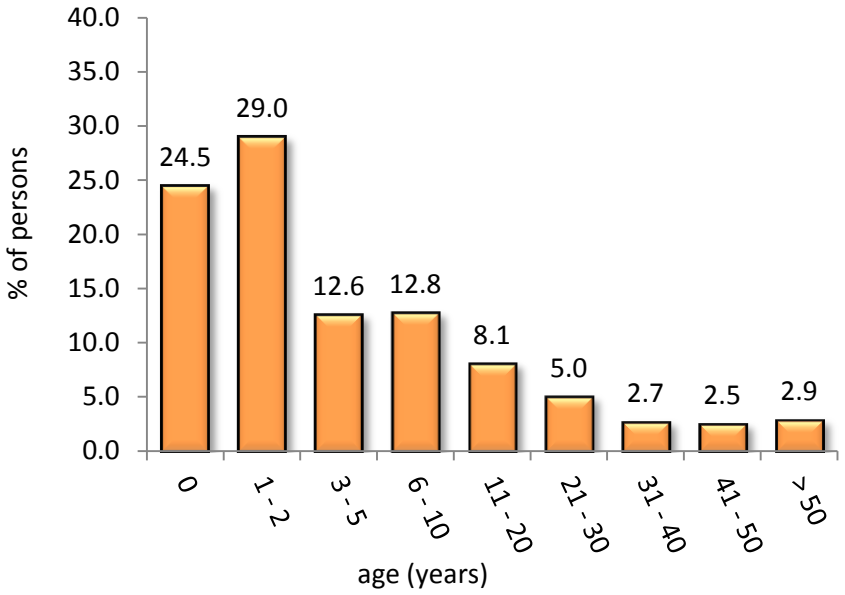
Part A.1

# Demographic characteristics of children with haemophilia

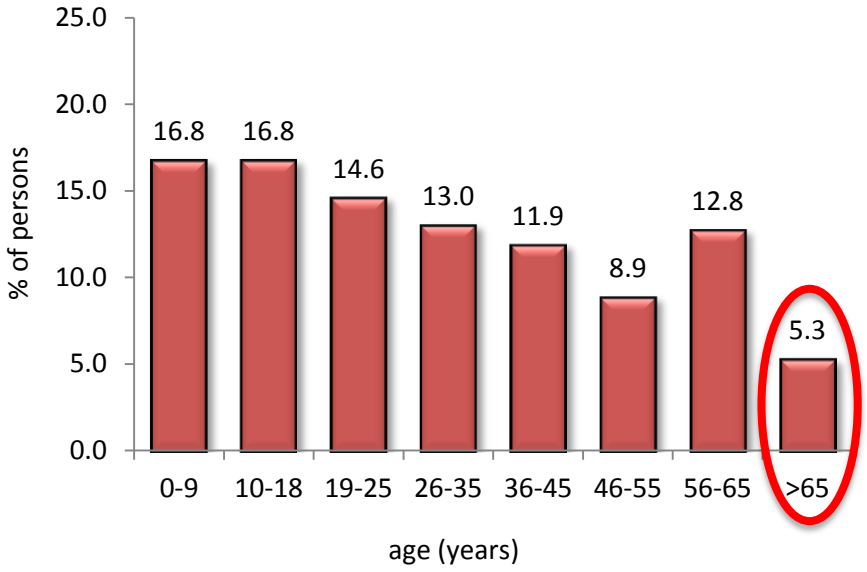
# Age

All
N=698

| | Age at diagnosis (years) |
|---|---|
| **N** | *556** |
| **Mean** | *8.1* |
| **Median (min - max)** | *2 (0 – 81)* |




| | Current age (years) |
|---|---|
| **N** | *698* |
| **Mean** | *31.2* |
| **Median (min - max)** | *27 (0 – 92)* |

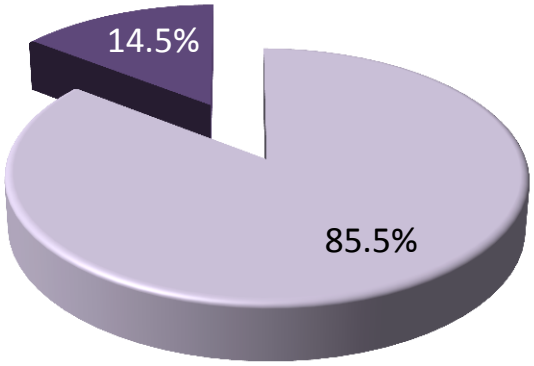



** Missing information on year of diagnosis in 142 persons.*

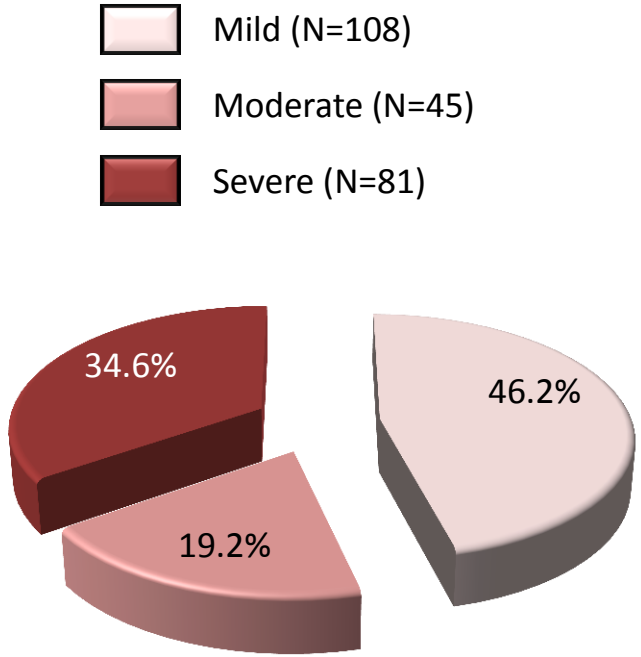
# Type and severity of haemophilia I

Children
N=234

**Type of haemophilia**




**Severity of haemophilia**

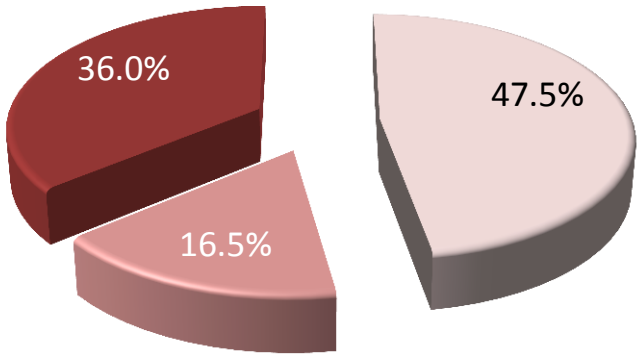


# Type and severity of haemophilia II

Children
N=234

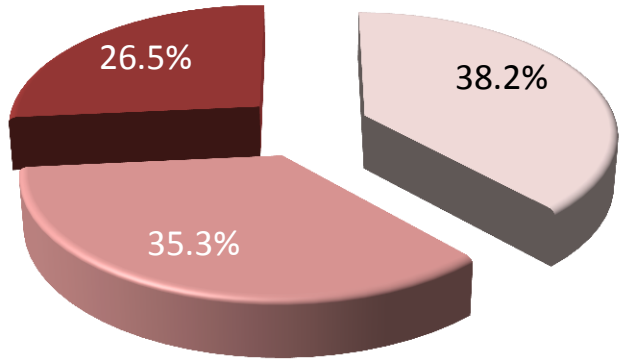
Haemophilia A (N=200)

- Mild (N=95)
- Moderate (N=33)
- Severe (N=72)



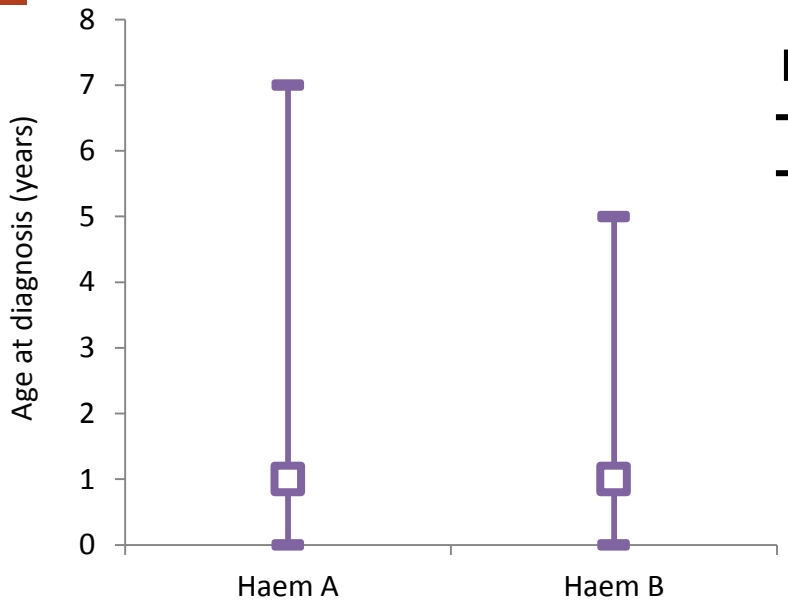

Haemophilia B (N=34)

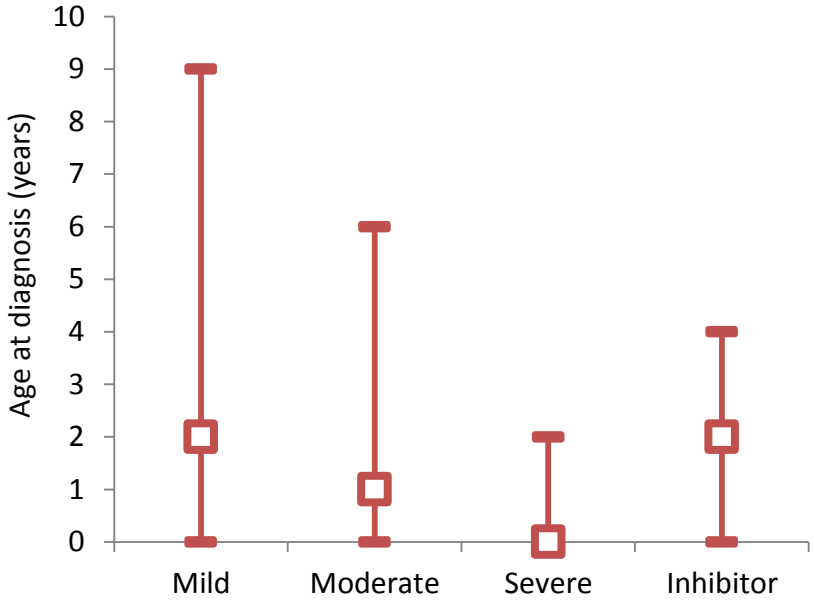
- Mild (N=13)
- Moderate (N=12)
- Severe (N=9)



# Age at diagnosis according to type and severity of haemophilia

Children
N=199[1]



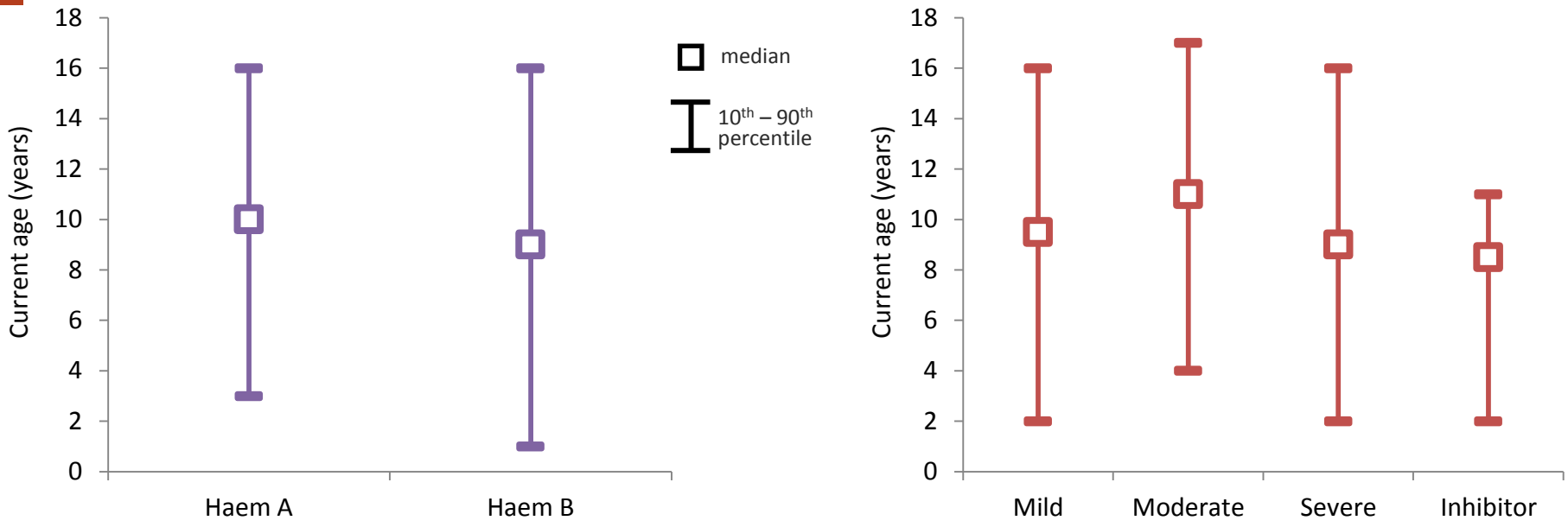

| Haemophilia A | Haemophilia B | Age at diagnosis (years) | Mild* | Moderate* | Severe* | Inhibitor+ |
|---|---|---|---|---|---|---|
| *166* | *33* | **N valid** | *93* | *37* | *69* | *6* |
| *2.3* | *2.0* | **Mean** | *3.5* | *2.0* | ***0.7*** | *1.5* |
| *1 (0 – 12)* | *1 (0 –13)* | **Median (min – max)** | *2 (0 – 13)* | *1 (0 – 12)* | ***0** (0 – 6)* | *1.5 (0 – 4)* |

*[1] Missing information on year of diagnosis in 35 children.*

** including persons with inhibitor*
*[+] in 2014*

# Actual age according to type and severity of haemophilia

Children
N=234




| Haemophilia A | Haemophilia B | Current age* (years) | Mild* | Moderate* | Severe* | Inhibitor+ |
|---|---|---|---|---|---|---|
| *200* | *34* | **N** | *108* | *45* | *81* | *6* |
| *9.6* | *8.8* | **Mean** | ***9.5*** | ***10.4*** | ***9.0*** | ***6.5*** |
| *10 (0 – 18)* | *9 (0 – 18)* | **Median (min – max)** | *9.5 (0 – 18)* | *11 (0 – 18)* | *9 (0 – 18)* | *6.5 (2 – 11)* |

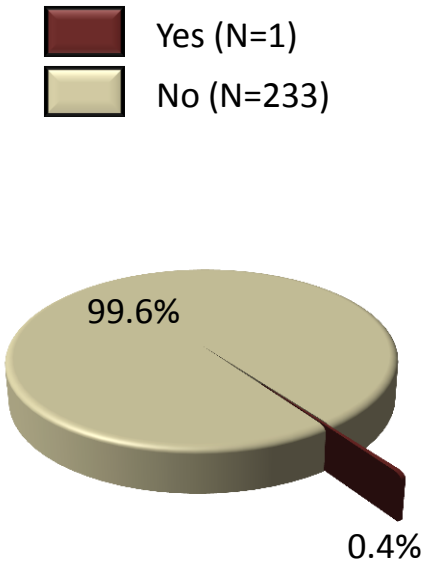
**Current age = age reached in year 2014*

** including persons with inhibitor*
*+ in 2014*

# Hepatitis experienced

Children
N=234

**Experienced hepatitis**




N=1

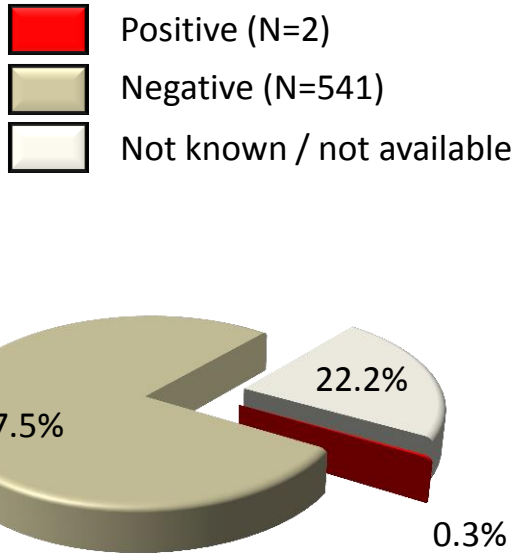
*One child has hepatitis C.*

*Data from last annual report of each person.*

# HIV

All
N=698

**HIV**




N=2 (+ 1 in another centre)

*All HIV-positive persons are adults.*

*Data from last annual report of each person.*

Part A.2

# Persons with haemophilia with inhibitor

All
N=698

# Persons with haemophilia with inhibitors in year 2014

- Active inhibitors were recorded in **12 persons** in year 2014 (+ 6 in another centre)
  - **6 children** and 6 adults
  - 11 haemophilia A and 1 heamophilia B
  - 9 severe, 1 moderate and 2 mild haemophilia
  - 10 HR and 2 LR
  - 4 patients were treated with rFVIIa, 2 patients with aPCC, other 2 patients with both rFVIIa and aPCC
  - 2 patients were without „by-pass“ therapy and 2 patients were without any recorded treatment
- ITT
  - Two of above mentioned 12 persons (1 child, 1 adult) finished ITT in 2014 as „unsuccessful“ (adult PWH started in 2014, child in 2011)
  - Two children started ITT in 2013 and continued in 2014
  - ITT was successfully finished in another 1 child during 2014, this child is inhibitor free now

All
N=10

# Persons with inhibitor

| | 1 | 2 | 3 | 4 | 5 | 6 | 7 | 8 | 9 | 10 | 11 | 12 | 13 |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| Age group | **child** | **child** | **child** | **child** | **child** | **child** | adult | adult | adult | adult | adult | adult | child |
| Year of birth | **2012** | **2011** | **2008** | **2007** | **2004** | **2003** | 1988 | 1977 | 1971 | 1956 | 1949 | 1941 | 2013 |
| Type of haemophilia | **A** | **A** | **A** | **B** | **A** | **A** | A | A | A | A | A | A | A |
| Severity | **sev** | **sev** | **sev** | **sev** | **sev** | **sev** | mild | sev | sev | sev | mild | mod | sev |
| Year of inhibitor development | **2014** | **2012** | **2013** | **2009** | **2009** | **2005** | 2013 | 2001 | 1988 | 1972 | 2012 | 2013 | 2011 |
| HR/LR | **HR** | **HR** | **HR** | **HR** | **HR** | **HR** | LR | HR | HR | HR | LR | HR | LR |
| „By-pass“ treatment in 2014 | **w/o by-pass therapy** | **-** | **-** | **rFVIIa** | **rFVII + aPCC** | **rFVIIa** | w/o any treatment | w/o any treatment | rFVII + aPCC | aPCC | aPCC | rFVIIa | - |
| ITT | **Planed for 2015** | **Since 2013** | **Since 2013** | - | **2011-2014, unsuc-cessful** | - | - | - | - | - | - | in 2014, unsuc-cessful | **2013-2014, successful** |

Part A.3

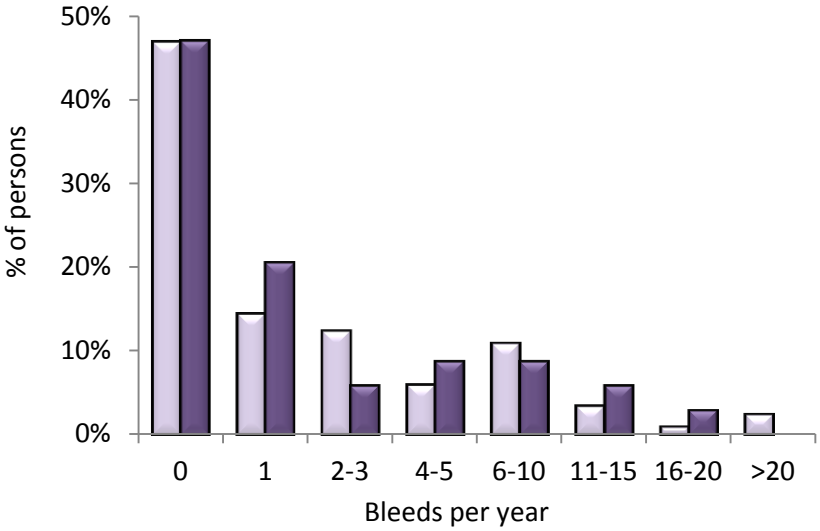
# Treatment outcomes including bleeding frequency in children with haemophilia 2014 data

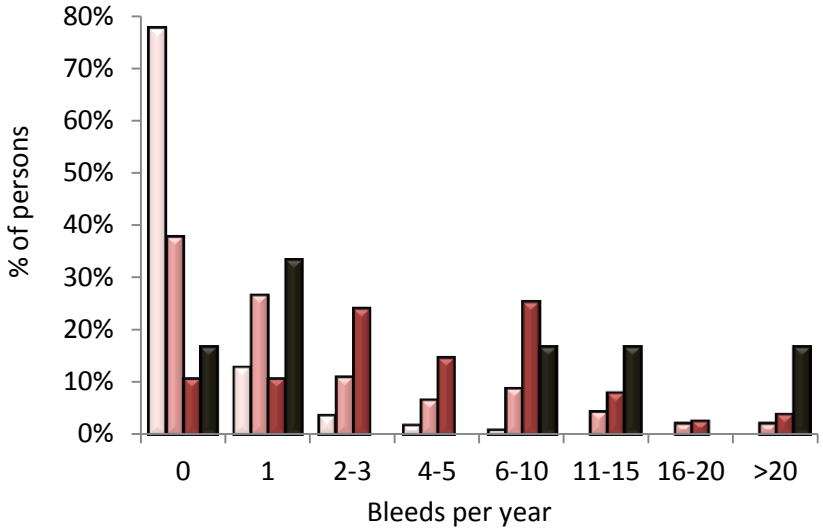
# Data from year 2014 – sample size

| | Valid persons | | | Persons with annual report in 2014 | | | Persons examined in 2014 | | | Persons treated in 2014 | |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | N | % | | N | % | | N | % | | N | % |
| All | 698 | 100% | → | 692 | 99.1% | → | 536 | 76.8% | → | 402 | 57.6% |
| of them with inhibitor | | | | 12 | | | 9 | | | 9 | |
| **Children** | **234** | **100%** | → | **231** | **98.7%** | → | **211** | **90.2%** | → | **138** | **59.0%** |
| **of them with inhibitor** | | | | **6** | | | **6** | | | **6** | |
| Adults | 464 | 100% | → | 461 | 99.4% | → | 325 | 70.0% | → | 264 | 56.9% |
| of them with inhibitor | | | | 6 | | | 3 | | | 3 | |

# Frequency of bleeding requiring treatment in 2014

Children
N=231




| Haemophilia A | Haemophilia B | Frequency of bleeding | Mild* | Moderate* | Severe* | Inhibitor |
|---|---|---|---|---|---|---|
| *197* | *34* | **N valid** | *105* | *45* | *75* | *6* |
| *2.8* | *2.7* | **Mean** | *0.4* | *3.0* | *5.7* | *8.2* |
| *1 (0 – 31)* | *1 (0 – 18)* | **Median (min – max)** | *0 (0 – 9)* | *1 (0 – 21)* | *4 (0 – 31)* | *4 (0 – 27)* |

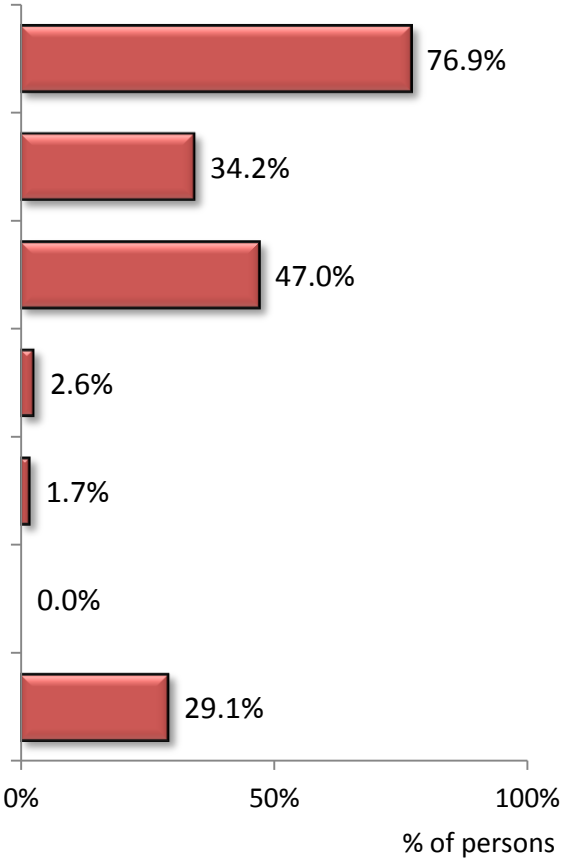
** without inhibitor*

# Location of bleeds in 2014

Children
N=231

121 (52.4%) children experienced bleeding requiring treatment at least once in year; 651 bleeds were recorded in total, 28 bleeds required hospitalization.
117 of these 121 children have recorded location of their bleeds. Localization is not known in 4 children.
110 (47.6%) children recorded no bleed during year 2014.

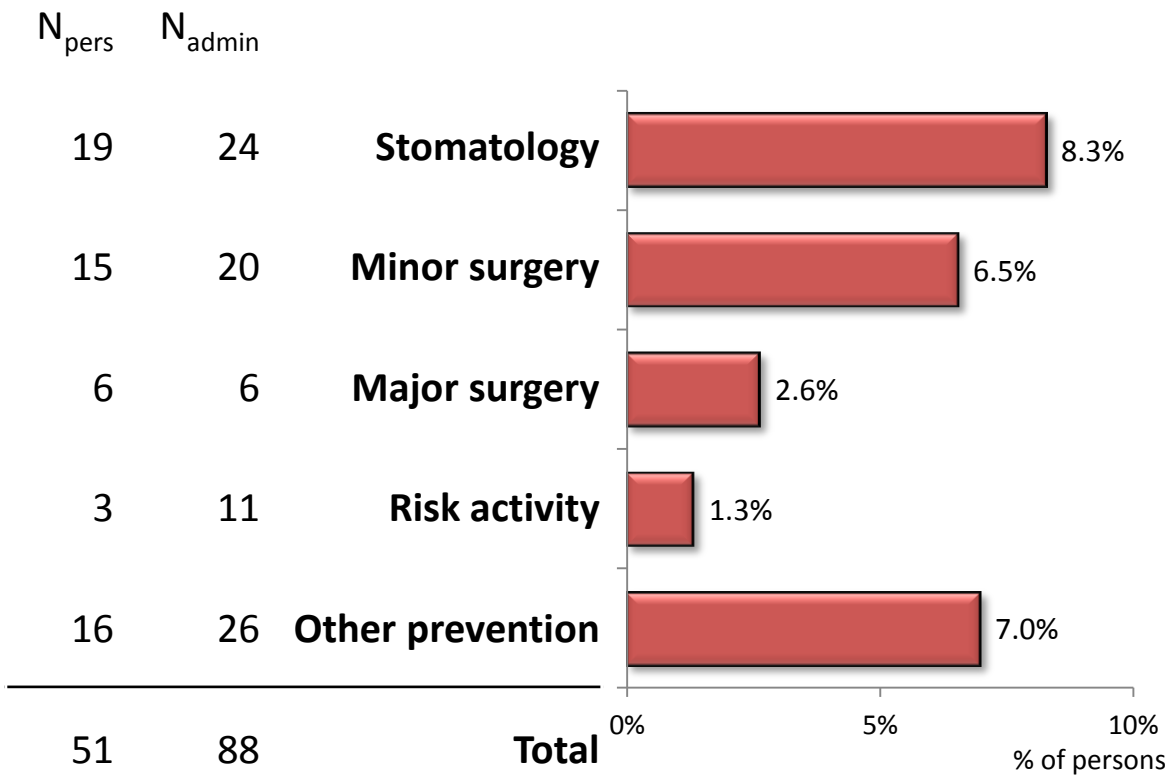
| $N_{pers}$ | $N_{bleeds}$ | |
|---|---|---|
| 90 | 319 | **Joints** |
| 40 | 104 | **Muscles** |
| 55 | 131 | **Subcutaneous** |
| 3 | 4 | **Urogenital tract** |
| 2 | 2 | **GIT** |
| 0 | 0 | **CNS** |
| 34 | 90 | **Other** |
| 117 | 653 | **Total** |



# Preventive administration in 2014

Children
N=231

51 (22.2%) children were given factor to prevent bleeding during/before risk situation.
88 preventive administrations were recorded in total.

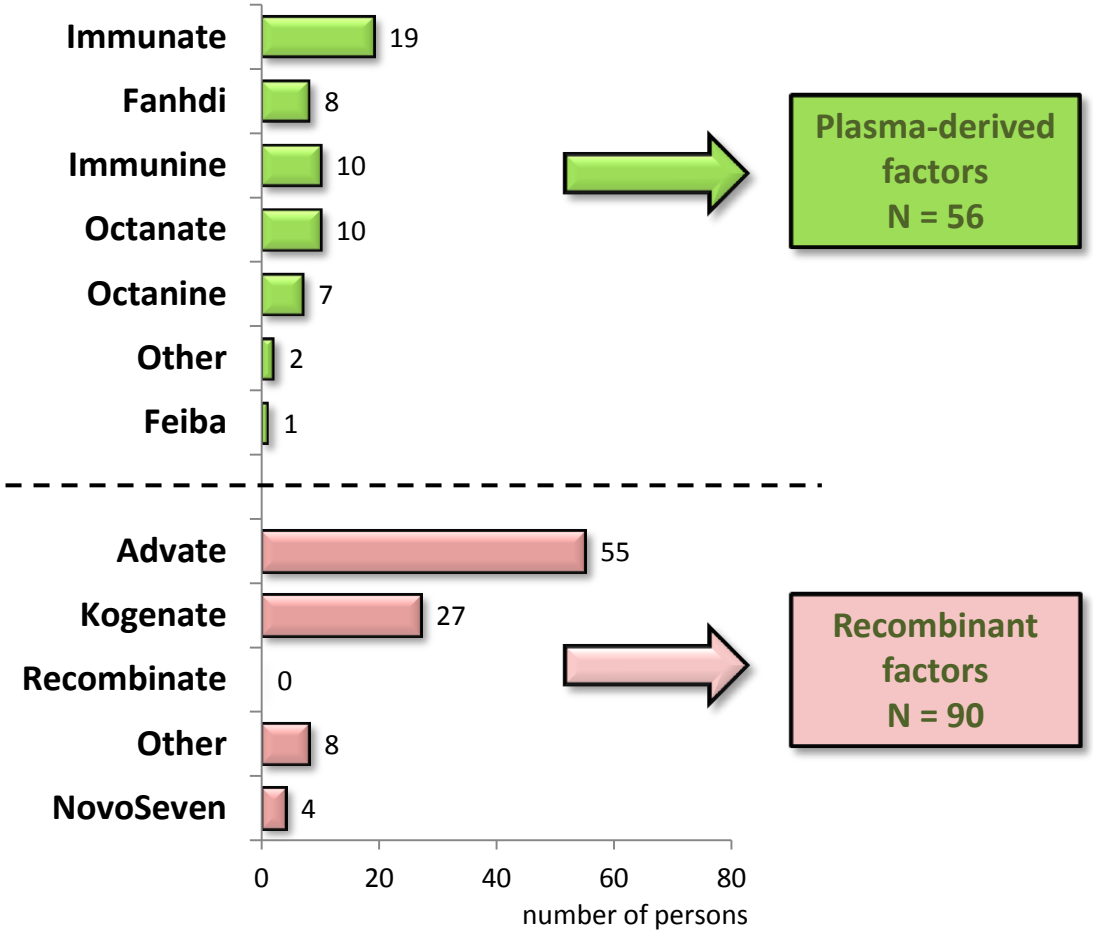


Part A.4

# Treatment data and factor consumption 2014 data

# Treatment

Children
N=138




138 children (60% of all children) received factor concentrates in 2014 (12 of them received more than one type/make of concentrate).
Plasma-derived factors were administered in 56 children (24.3% of all, 40.6% of treated children), recombinant factors in 90 children (39.1% of all, 65.2% of treated children).
Eight children were treated with both plasma-derived and recombinant factor.

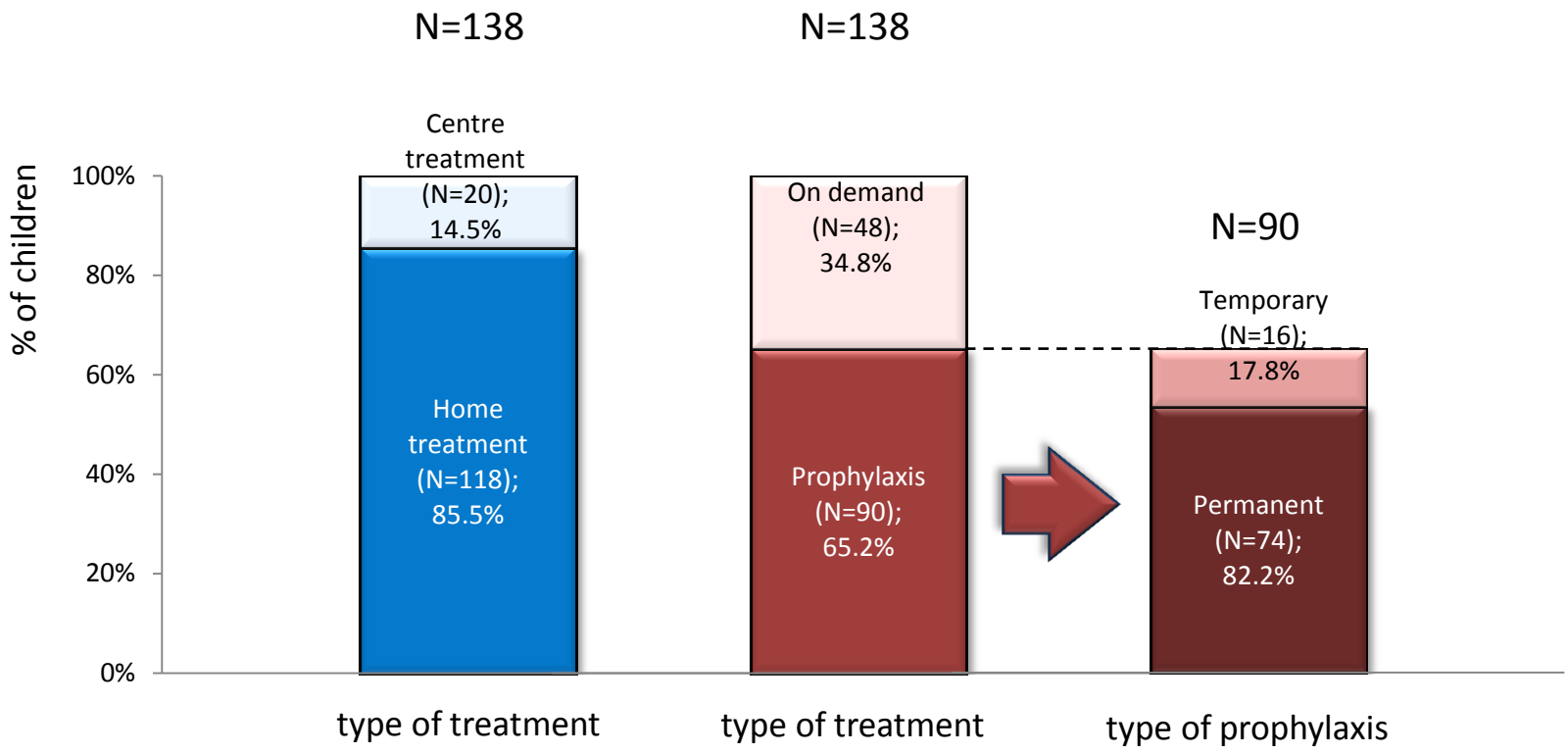
# Comparison of treatment in years 2013 and 2014

Children
N=231

| | 2014 | | | 2013 | | |
|---|---|---|---|---|---|---|
| | N | % of all PWHs | % treated PWHs | N | % of all PWHs | % treated PWHs |
| **All children with treatment** | 138 | 59.7 | 100.0 | 139 | 60.7 | 100.0 |
| ***Plasma-derived factor*** | *52* | *22.5* | *37.7* | *66* | *28.8* | *47.5* |
| ***Recombinant factor*** | ***86*** | ***37.2*** | ***62.3*** | ***73*** | ***31.9*** | ***52.5*** |
| **Without treatment** | 93 | 40.3 | - | 90 | 39.3 | - |
| **Total** | 231 | 100.0 | - | 229 | 100.0 | - |

# Type of treatment

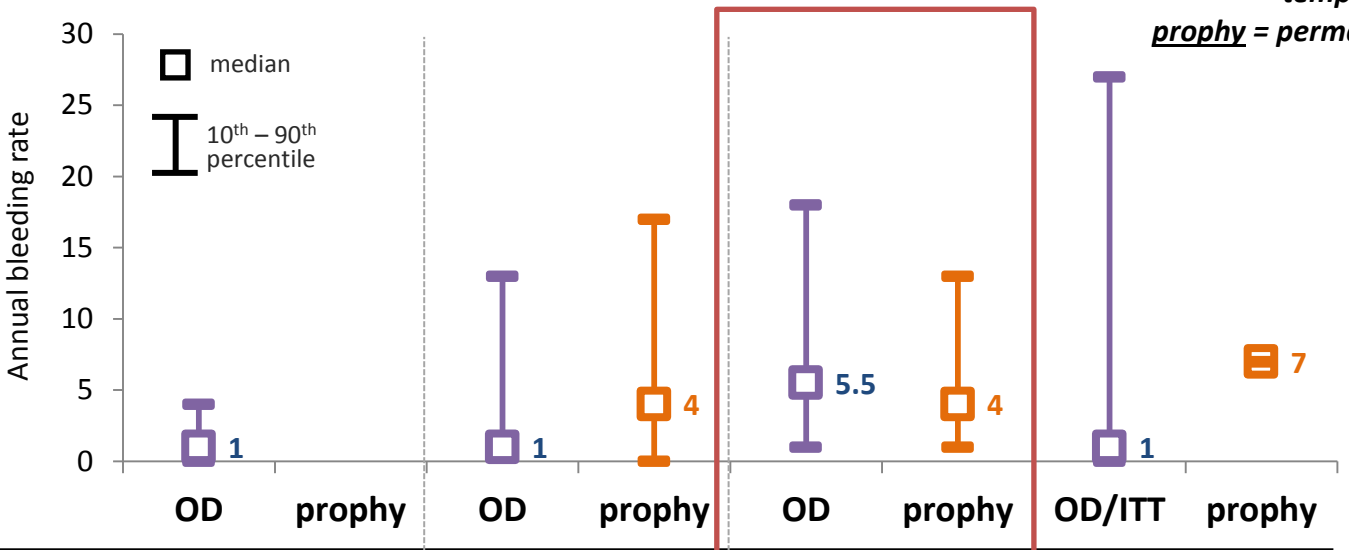
Children
N=138



# Annual bleeding rate according to treatment regimen

Children
N=138

***Treatment regimen:***
***<u>OD</u> = on demand and/or temporary prophylaxis***
***<u>prophy</u> = permanent prophylaxis***




| Frequency of bleeding | Mild* | | Moderate* | | Severe* | | Inhibitor | |
|---|---|---|---|---|---|---|---|---|
| N total | 28 | 0 | 23 | 9 | 8 | 64 | 5 | 1 |
| Mean | 1.4 | | 3.8 | 5.0 | 6.6 | 5.9 | 8.4 | 7.0 |
| Median (min – max) | 0 (0 – 9) | | 1 (0 – 21) | 4 (0 – 17) | 5.5 (1 – 18) | 4 (0 – 31) | 1 (0 – 27) | 7 |
| Total no of recorded bleeds | 39 | | 88 | 45 | 53 | 377 | 42 | 7 |
| children on permanent prophylaxis | 0 (0%) | | 9 (28.1%) | | 64 (88.9%) | | 1 (16.7%) | |
| % of drugs (FVIII and FIX) consumed by children on permanent prophylaxix | 0 % | | 78.6 % | | 96.9 % | | 37.1 % | |

* without inhibitor

# Joint and other bleeds according to treatment regimen

Children N=134[1]

| Frequency of bleeding | Mild* | | Moderate* | | Severe* | | Inhibitor | |
|---|---|---|---|---|---|---|---|---|
| Treatment regimen | *OD* | *prophy* | *OD* | *prophy* | *OD* | *prophy* | *OD/ITT* | *prophy* |
| N valid | *26* | *0* | *22* | *9* | *7* | *64* | *5* | *1* |
| **JOINT BLEEDS** | | | | | | | | |
| Mean | *0.6* | | *1.6* | *3.0* | ***3*** | ***3.0*** | *5.2* | *2.0* |
| Median (range) | *0 (0 – 3)* | | *0 (0 – 8)* | *1 (0 – 13)* | ***2 (0 – 7)*** | ***2 (0 – 13)*** | *1 (0 – 16)* | *2* |
| Total no of recorded bleeds | *15* | | *34* | *27* | ***21*** | ***194*** | *26* | *2* |
| **OTHER BLEEDS** | | | | | | | | |
| Mean | *0.8* | | *2.6* | *1.9* | ***4.4*** | ***2.9*** | *3.4* | *5.0* |
| Median (range) | *0 (0 – 6)* | | *1.5 (0 – 13)* | *1 (0 – 7)* | ***3 (0 – 11)*** | ***2 (0 – 31)*** | *1 (0 – 12)* | *5* |
| Total no of recorded bleeds | *20* | | *58* | *17* | ***31*** | ***183*** | *17* | *5* |


** without inhibitor*

*[1] missing location of bleeds in 4 children*

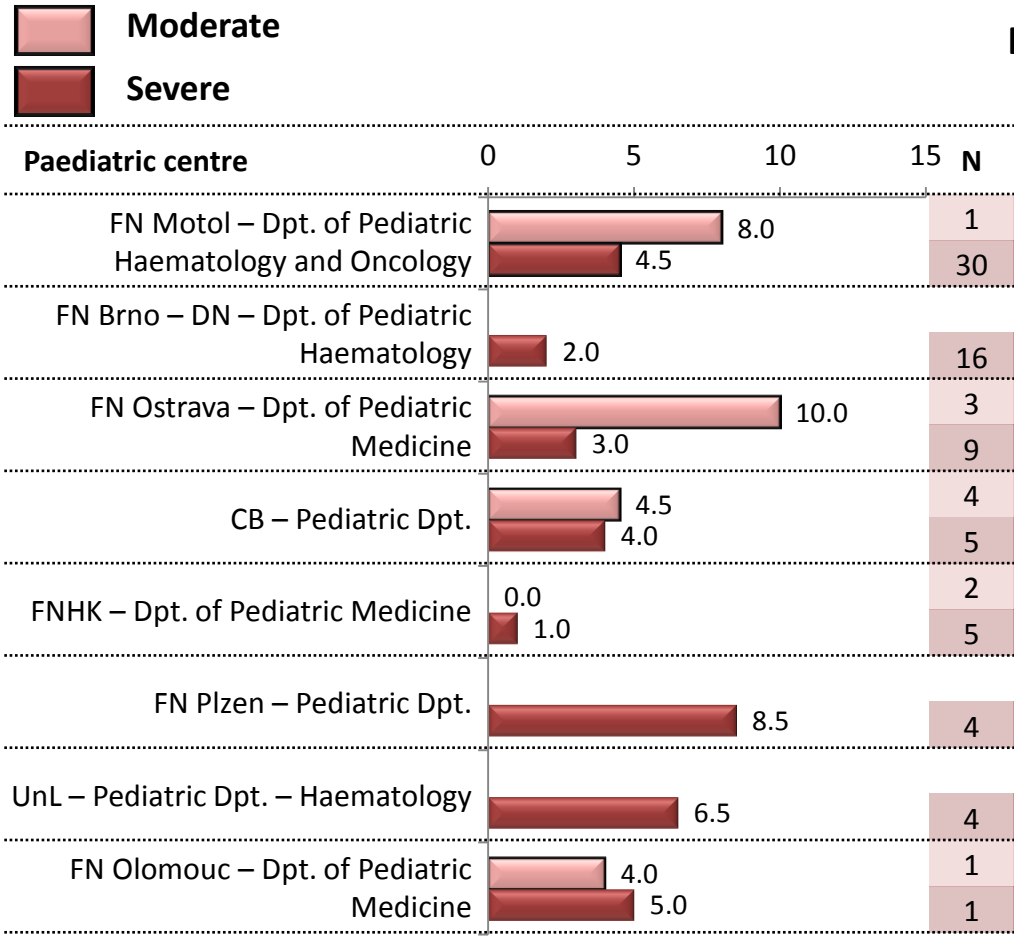
***Treatment regimen:***

***<u>OD</u> = on demand and/or temporary prophylaxis***

***<u>prophy</u> = permanent prophylaxis***

# Annual bleeding rate on permanent prophylaxis in paediatric centres

PWHs on prophy in paed. centres N=85




**Frequency of bleeding in PWHs without inhibitor on permanent prophylaxis**

| Paediatric centre | N | Mean | Median | Min | Max | Severity |
|---|---|---|---|---|---|---|
| FN Motol – Dpt. of Pediatric Haematology and Oncology | 1 | 8.0 | **8.0** | 8 | 8 | Moderate |
| | 30 | 5.5 | **4.5** | 0 | 16 | Severe |
| FN Brno – DN – Dpt. of Pediatric Haematology | 16 | 4.2 | **2.0** | 0 | 21 | Severe |
| FN Ostrava – Dpt. of Pediatric Medicine | 3 | 9.3 | **10.0** | 1 | 17 | Moderate |
| | 9 | 6.6 | **3.0** | 2 | 27 | Severe |
| CB – Pediatric Dpt. | 4 | 3.8 | **4.5** | 1 | 5 | Moderate |
| | 5 | 4.2 | **4.0** | 1 | 7 | Severe |
| FNHK – Dpt. of Pediatric Medicine | 2 | 0.0 | **0.0** | 0 | 0 | Moderate |
| | 5 | 1.8 | **1.0** | 0 | 4 | Severe |
| FN Plzen – Pediatric Dpt. | 4 | 12.5 | **8.5** | 2 | 31 | Severe |
| UnL – Pediatric Dpt. – Haematology | 4 | 6.3 | **6.5** | 2 | 10 | Severe |
| FN Olomouc – Dpt. of Pediatric Medicine | 1 | 4.0 | **4.0** | 4 | 4 | Moderate |
| | 1 | 5.0 | **5.0** | 5 | 5 | Severe |

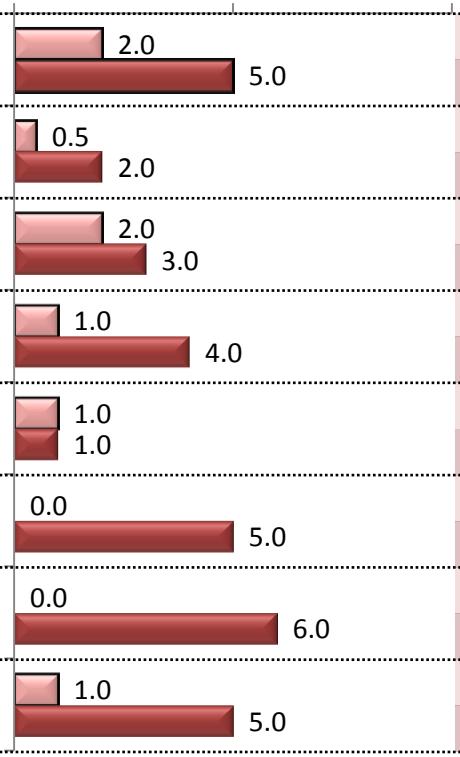
# Annual bleeding rate regardless prophylaxis in paediatric centres

PWHs in paed. centres N=140

Moderate

Severe

**Frequency of bleeding in PWHs without inhibitor regardless of prophylaxis**




| Paediatric centre | N | Mean | Median | Min | Max | % on permanent prophylaxis |
|---|---|---|---|---|---|---|
| FN Motol – Dpt. of Pediatric Haematology and Oncology | 14 | 4.4 | **2.0** | 0 | 21 | **7.1** |
| | 40 | 6.0 | **5.0** | 0 | 18 | **75.0** |
| FN Brno – DN – Dpt. of Pediatric Haematology | 6 | 0.7 | **0.5** | 0 | 2 | **0.0** |
| | 17 | 3.8 | **2.0** | 0 | 21 | **94.1** |
| FN Ostrava – Dpt. of Pediatric Medicine | 9 | 5.9 | **2.0** | 0 | 17 | **33.3** |
| | 10 | 5.9 | **3.0** | 0 | 27 | **90.0** |
| CB – Pediatric Dpt. | 7 | 2.3 | **1.0** | 0 | 5 | **57.1** |
| | 5 | 4.2 | **4.0** | 1 | 7 | **100.0** |
| FNHK – Dpt. of Pediatric Medicine | 7 | 1.0 | **1.0** | 0 | 3 | **28.6** |
| | 5 | 1.8 | **1.0** | 0 | 4 | **100.0** |
| FN Plzen – Pediatric Dpt. | 1 | 0.0 | **0.0** | 0 | 0 | **0.0** |
| | 5 | 10.2 | **5.0** | 1 | 31 | **80.0** |
| UnL – Pediatric Dpt. – Haematology | 2 | 0.0 | **0.0** | 0 | 0 | **0.0** |
| | 5 | 5.2 | **6.0** | 1 | 10 | **80.0** |
| FN Olomouc – Dpt. of Pediatric Medicine | 3 | 2.0 | **1.0** | 1 | 4 | **33.3** |
| | 2 | 5.0 | **5.0** | 5 | 5 | **50.0** |

# Prophylactic regimens and tretament outcomes in paediatric centres

PWHs in paed. centres N=140

| Paediatric centre | Severity | Total N | % on perm prophy | % w/o perm prophy | Dosing of prophylaxis (IU/kg per week) | | | | | Annual bleeding rate ON permanent prophylaxis | | Annual bleeding rate WITHOUT perm prophy | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| | | | | | N | Mean | Median | Min | Max | Mean | Median | Mean | Median |
| FN Motol | **Moderate** | 14 | **7.1** | 92.9 | 1 | 90.6 | **90.6** | | | 8.0 | **8.0** | 4.2 | 2.0 |
| | **Severe** | 40 | **75.0** | 25.0 | 30 | 81.4 | **81.0** | 45.1 | 148.9 | 5.5 | **4.5** | 8.1 | 7.5 |
| FN Brno – DN | **Moderate** | 6 | **0.0** | 100.0 | - | | | | | - | | 0.8 | 1.0 |
| | **Severe** | 17 | **94.1** | 5.9 | 16 | 65.1 | **67.7** | 22.5 | 115.1 | 4.2 | **2.0** | 0.5 | 0.5 |
| FN Ostrava – Ped. Dpt. | **Moderate** | 9 | **33.3** | 66.7 | 3 | 57.0 | **46.0** | 41.7 | 83.3 | 9.3 | **10.0** | 4.2 | 1.0 |
| | **Severe** | 10 | **90.0** | 10.0 | 9 | 83.5 | **75.0** | 48.3 | 130.4 | 6.6 | **3.0** | 0.0 | 0.0 |
| CB – Ped. Dpt. | **Moderate** | 7 | **57.1** | 42.9 | 4 | 53.3 | **52.6** | 31.3 | 76.9 | 3.8 | **4.5** | 0.3 | 0.0 |
| | **Severe** | 5 | **100.0** | 0.0 | 5 | 64.3 | **61.5** | 49.8 | 88.2 | 4.2 | **4.0** | - | |
| FNHK – Ped. Dpt. | **Moderate** | 7 | **28.6** | 71.4 | 0 | na | | | | 0.0 | **0.0** | 2.0 | 2.0 |
| | **Severe** | 5 | **100.0** | 0.0 | 0 | na | | | | 1.8 | **1.0** | - | |
| FN Plzen – Ped. Dpt. | **Moderate** | 1 | **0.0** | 100.0 | - | - | | | | - | | 0.0 | 0.0 |
| | **Severe** | 5 | **80.0** | 20.0 | 4 | 46.2 | **48.8** | 22.7 | 64.2 | 12.5 | **8.5** | 1.0 | 1.0 |
| UnL – Ped. Dpt. | **Moderate** | 2 | **0.0** | 100.0 | - | - | | | | - | | 0.0 | 0.0 |
| | **Severe** | 5 | **80.0** | 20.0 | 4 | 45.8 | **39.9** | 17.9 | 85.7 | 6.3 | **6.5** | 1.0 | 1.0 |
| FN Olomouc – Ped. Dpt. | **Moderate** | 3 | **33.3** | 66.7 | 1 | 34.5 | **34.5** | | | 4.0 | **4.0** | 1.0 | 1.0 |
| | **Severe** | 2 | **50.0** | 50.0 | 0 | na | | | | 5.0 | **5.0** | na | |

All

# Consumption of drugs

| | Drug (IU) | Total annual consumption | Number of treated persons | Average annual consumption per treated person | Number of valid persons | Average annual consumption per valid person |
|---|---|---|---|---|---|---|
| FVIII | Immunate | 8 858 600 | 116 | 76 367.2 | 602 | 14 715.3 |
| | Fanhdi | 6 622 350 | 78 | 84 901.9 | | 11 000.6 |
| | Octanate | 2 304 000 | 20 | 115 200.0 | | 3 827.2 |
| | Other plasma-derived | 2 130 850 | 7 | 304 407.1 | | 3 539.6 |
| | Advate | 6 517 104 | 74 | 88 069.0 | | 10 825.8 |
| | Kogenate | 3 950 750 | 43 | 91 877.9 | | 6 562.7 |
| | Recombinate | 1 817 500 | 23 | 79 021.7 | | 3 019.1 |
| | Other recombinant | 1 885 050 | 9 | 209 450.0 | | 3 131.3 |
| | **FVIII total (IU)** | **34 086 204** | **340** | **100 253.5** | | **56 621.6** |
| FIX | Immunine | 1 892 700 | 36 | 52 575.0 | 96 | 19 715.6 |
| | Octanine | 2 922 000 | 20 | 146 100.0 | | 30 437.5 |
| | Other recombinant | 600 820 | 4 | 150 205.0 | | 6 258.5 |
| | **FIX total (IU)** | **5 415 520** | **58** | **93 371.0** | | **56 411.7** |
| aPCC | Feiba | 252 000 | 4 | 63 000.0 | | |
| rFVIIa | NovoSeven (mg) | 1 715 mg | 6 | 285.8 mg | | |
| **Plasma-derived factors - TOTAL*** | | **24 730 500** | **266** | **92 971.8** | 698 | **35 430.5** |
| **Recombinant factors - TOTAL*** | | **14 771 224** | **145** | **101 870.5** | | **21 162.2** |
| **TOTAL CONSUMPTION (IU)*** | | **39 501 724** | **398** | **99 250.6** | | **56 592.7** |

- plasma-derived factors = Immunate, Fanhdi, Octanate, Immunine, Octanine, Other plasma-derived
- recombinant factors = Advate, Kogenate, Recombinate, BAX 326, Other recombinant

*TOTAL CONSUMPTION = all mentioned drugs excluding Feiba and NovoSeven

Children

# Consumption of drugs

| | Drug (IU) | Total annual consumption | Number of treated persons | Average annual consumption per treated child | Number of valid persons | Average annual consumption per valid person |
|---|---|---|---|---|---|---|
| FVIII | Immunate | 1 110 500 | 19 | 58 447.4 | 200 | 5 552.5 |
| | Fanhdi | 1 215 000 | 8 | 151 875.0 | | 6 075.0 |
| | Octanate | 1 851 500 | 10 | 185 150.0 | | 9 257.5 |
| | Other plasma-derived | 181 000 | 2 | 90 500.0 | | 905.0 |
| | Advate | 4 356 604 | 55 | 79 211.0 | | 21 783.0 |
| | Kogenate | 1 605 250 | 27 | 59 453.7 | | 8 026.3 |
| | Recombinate | | | | | |
| | Other recombinant | 1 106 802 | 5 | 221 360.4 | | 5 534.0 |
| | **FVIII total (IU)** | **11 426 656** | **116** | **98 505.7** | | **57 133.3** |
| FIX | Immunine | 558 200 | 10 | 55 820.0 | 34 | 16 417.6 |
| | Octanine | 272 000 | 7 | 38 857.1 | | 8 000.0 |
| | Other recombinant | 309 534 | 3 | 103 178.0 | | 9 103.9 |
| | **FIX total (IU)** | **1 139 734** | **20** | **56 986.7** | | **33 521.6** |
| aPCC | Feiba | 5 000 | 1 | 5 000.0 | | |
| rFVIIa | NovoSeven (mg) | 1 338 mg | 4 | 334.5 mg | | |
| **Plasma-derived factors - TOTAL*** | | **5 188 200** | **55** | **94 330.9** | 234 | **22 171.8** |
| **Recombinant factors - TOTAL*** | | **7 378 190** | **86** | **85 792.9** | | **31 530.7** |
| **TOTAL CONSUMPTION (IU)*** | | **12 566 390** | **136** | **92 399.9** | | **53 702.5** |

- plasma-derived factors = Immunate, Fanhdi, Octanate, Immunine, Octanine, Other plasma-derived
- recombinant factors = Advate, Kogenate, Recombinate, BAX 326, Other recombinant

*TOTAL CONSUMPTION = all mentioned drugs excluding Feiba and NovoSeven

Adults

# Consumption of drugs

| | Drug (IU) | Total annual consumption | Number of treated persons | Average annual consumption per treated person | Number of valid persons | Average annual consumption per valid person |
|---|---|---|---|---|---|---|
| FVIII | Immunate | 7 748 100 | 97 | 79 877.3 | 402 | 19 273.9 |
| | Fanhdi | 5 407 350 | 70 | 77 247.9 | | 13 451.1 |
| | Octanate | 452 500 | 10 | 45 250.0 | | 1 125.6 |
| | Other plasma-derived | 1 949 850 | 5 | 389 970.0 | | 4 850.4 |
| | Advate | 2 160 500 | 19 | 113 710.5 | | 5 374.4 |
| | Kogenate | 2 345 500 | 16 | 146 593.8 | | 5 834.6 |
| | Recombinate | 1 817 500 | 23 | 79 021.7 | | 4 521.1 |
| | Other recombinant | 778 248 | 4 | 194 562.0 | | 1 935.9 |
| | **FVIII total (IU)** | **22 659 548** | **224** | **101 158.7** | | **56 367.0** |
| FIX | Immunine | 1 334 500 | 26 | 51 326.9 | 62 | 21 524.2 |
| | Octanine | 2 650 000 | 13 | 203 846.2 | | 42 741.9 |
| | Other recombinant | 291 286 | 1 | 291 286.0 | | 4 698.2 |
| | **FIX total (IU)** | **4 275 786** | **38** | **112 520.7** | | **68 964.3** |
| aPCC | Feiba | 247 000 | 3 | 82 333.3 | | |
| rFVIIa | NovoSeven (mg) | 1 715 mg | 6 | 285.8 mg | | |
| **Plasma-derived factors - TOTAL*** | | **19 542 300** | **211** | **92 617.5** | 464 | **42 117.0** |
| **Recombinant factors - TOTAL*** | | **7 393 034** | **59** | **125 305.7** | | **15 933.3** |
| **TOTAL CONSUMPTION (IU)*** | | **26 935 334** | **262** | **102 806.6** | | **58 050.3** |

- plasma-derived factors = Immunate, Fanhdi, Octanate, Immunine, Octanine, Other plasma-derived
- recombinant factors = Advate, Kogenate, Recombinate, BAX 326, Other recombinant

*TOTAL CONSUMPTION = all mentioned drugs excluding Feiba and NovoSeven

# Závěr

- **Léčba dětí s hemofilií je v ČR na dobré úrovni**
  - Medián 2 kloubní krvácení ročně
  - Jsou však i děti s 20 krváceními/rok
- Existuje  stále diskrepance mezi výsledky léčby v jednotlivých centrech
  - Která není dána (jen) dávkováním
- Cílem by mělo být dosáhnout nižšího počtu (nejen) kloubních krvácení ve všech centrech ČNHP
  - Multidisciplinární spolupráce  v rámci každého centra a snaha o  lepší compliance/adherence pacientů a rodin
  - Léčba šitá na míru s použitím farmakokinetiky
  - Udržení nízké incidence inhibitorů (20% PUPs s těžkou HA)
  - Pravidelné (sebe)hodnocení léčby objektivním způsobem v každém jednom centru i napříč centry ČNHP
    - Počty krvácení, HJHS a pod…

# Poděkování


- Všem lékařům, sestřičkám, datamanagerům a ostatním pracovníkům center PSDH/ČNHP
- IBA MU za zpracování dat
  - Především P. Ovesné ☺
- Poskytovatelům grantů ČNHP

- Podpořeno z dotačního programu MZ: Národní akční plány a koncepce