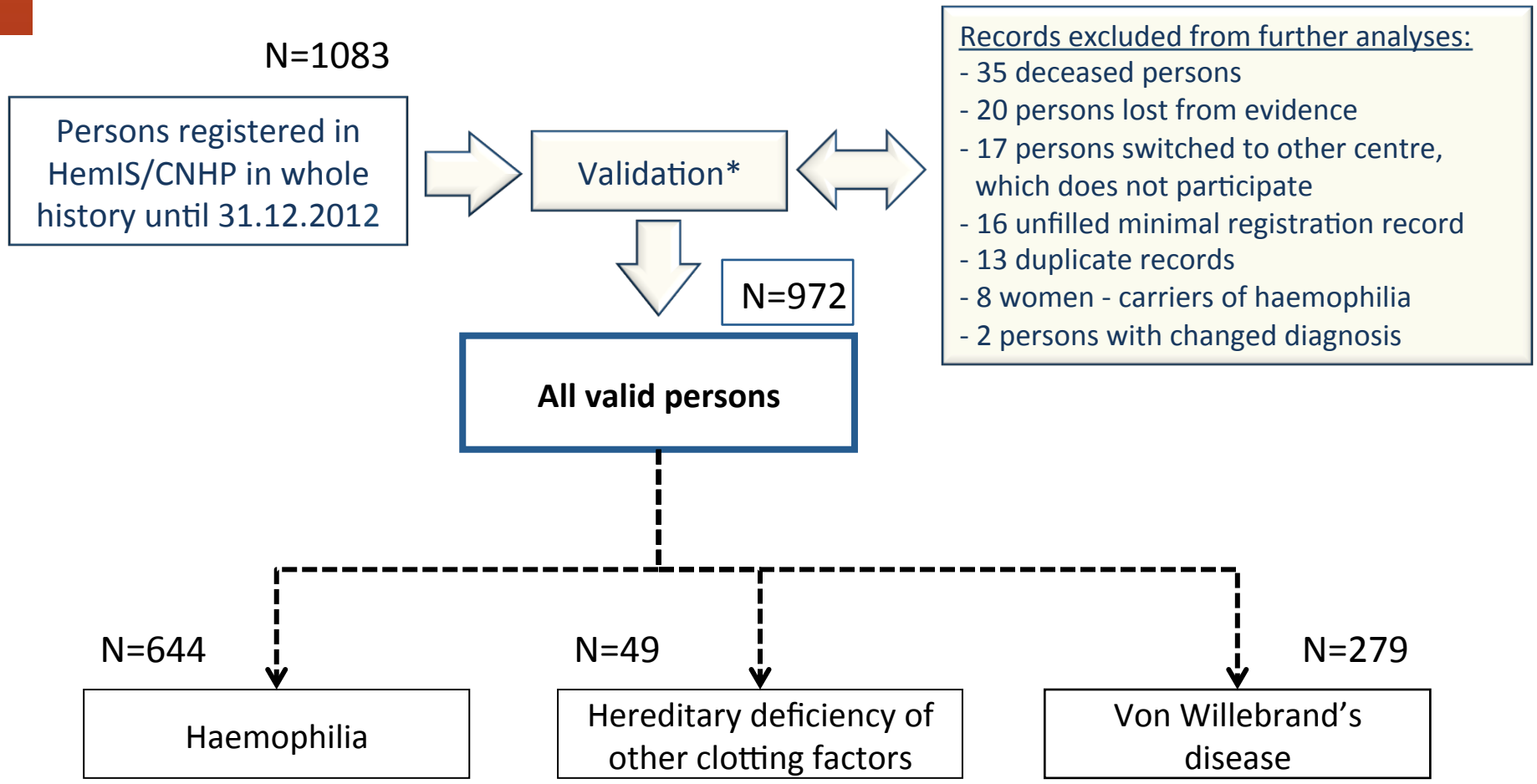
The current status of care for persons with haemophilia and von Willebrand's disease registered within CNHP registry

Jan Blatný, Petra Ovesná, Petr Brabec

on behalf of

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

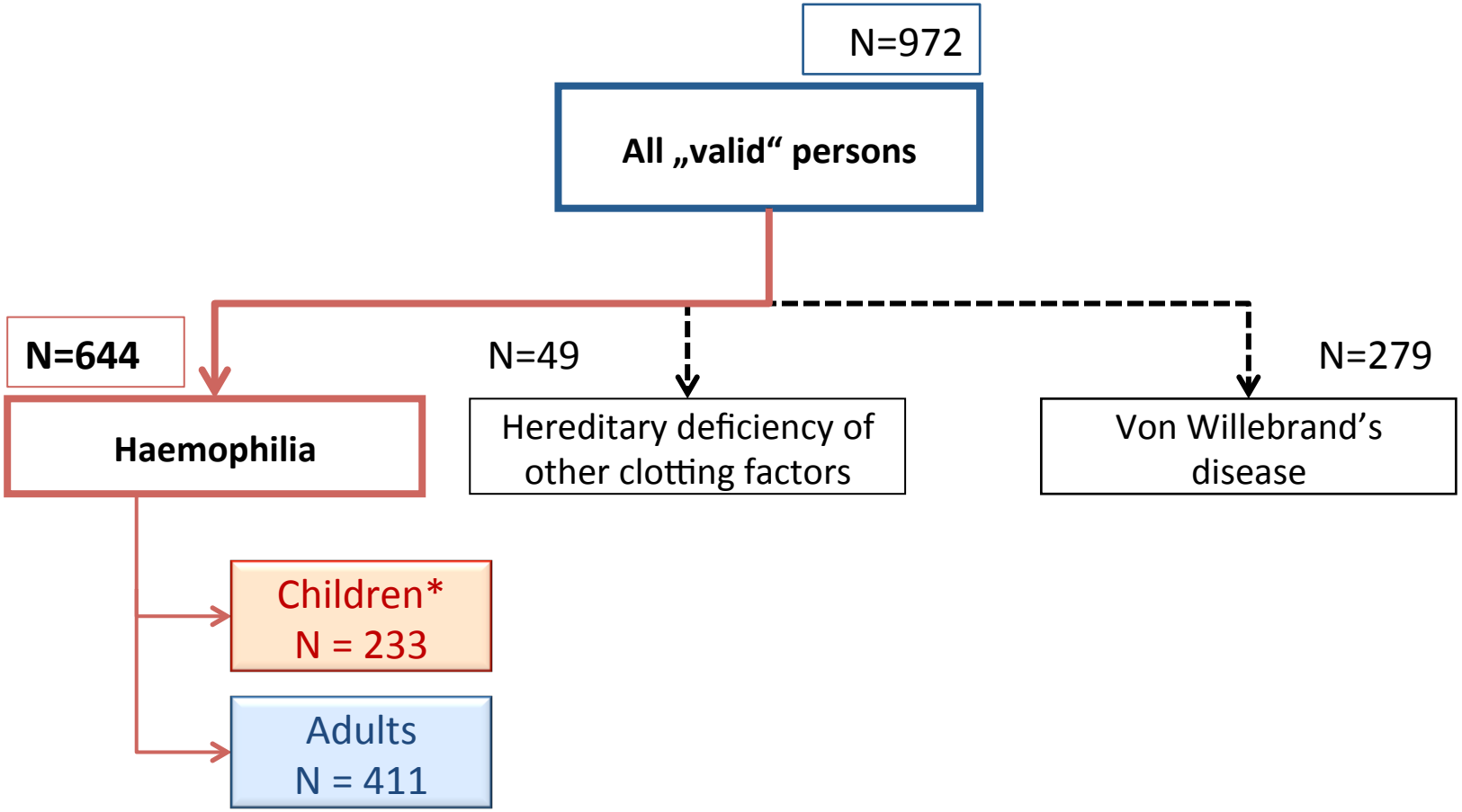
Sample size, valid records



Part A

Persons with haemophilia (PWH)

Sample size



* *Persons under 19 years old in 2012*

Participating centres in CNHP

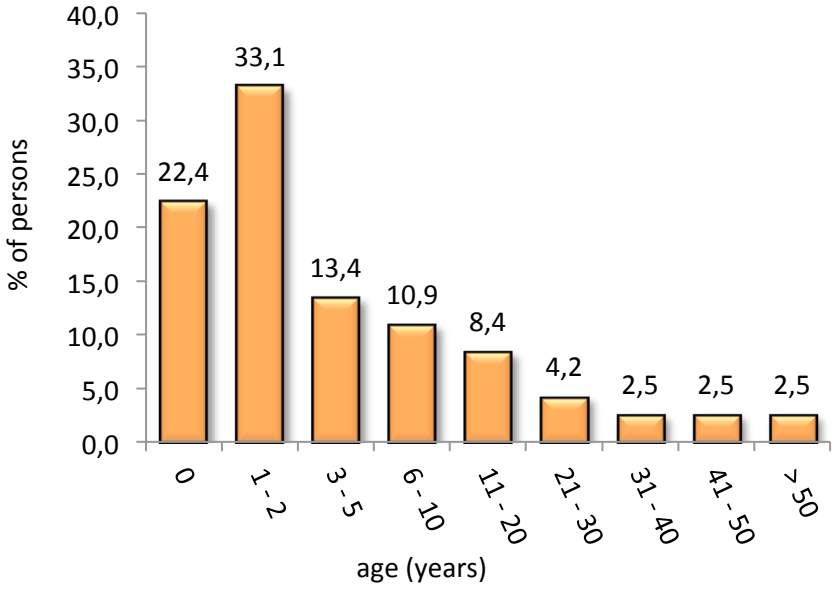
Paediatric centres	Valid persons N	%
FN Motol – Dpt. of Pediatric Haematology and Oncology	86	13.4
FN Brno – DN – Dpt. of Pediatric Haematology	46	7.1
FNHK – Dpt. of Pediatric Medicine	35	5.4
UnL – Pediatric Dpt. – Haematology	29	4.5
FN Ostrava – Dpt. of Pediatric Medicine	29	4.5
FN Olomouc – Dpt. of Pediatric Medicine	11	1.7
FN Plzen – Pediatric Dpt.	10	1.6
CB – Pediatric Dpt.	9	1.4

Adult centres	Valid persons N	%
FN Brno – OKH	124	19.3
FN Ostrava – Blood centre	74	11.5
FN Olomouc – Haemato-Oncology Dpt.	57	8.9
FN Plzen – UKBH	38	5.9
CB – OKH	26	4.0
FNHK – OKH	22	3.4
KN Liberec – OKH	20	3.1
UnL – OKH	8	1.2
Plzen – Health Centre – Haematology	7	1.1
UHKT Praha	6	0.9
Kolin – Haematology and Transfusion Dpt.	5	0.8
Pelhrimov - OHT	2	0.3

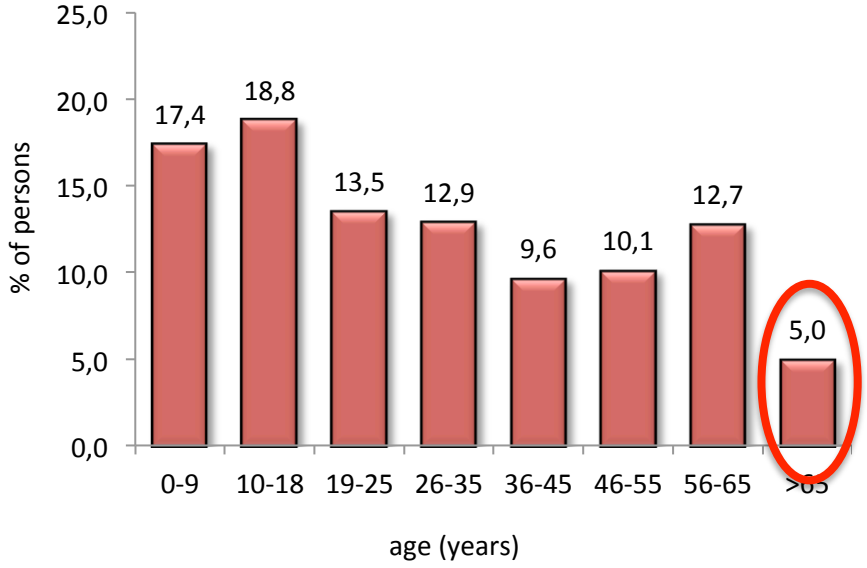
Age

All
N=644

	Age at diagnosis (years)
N	*477**
Mean	*7.6*
Median (min - max)	*2 (0 – 69)*



	Current age (years)
N	*644*
Mean	*30.5*
Median (min - max)	*26 (0 – 90)*

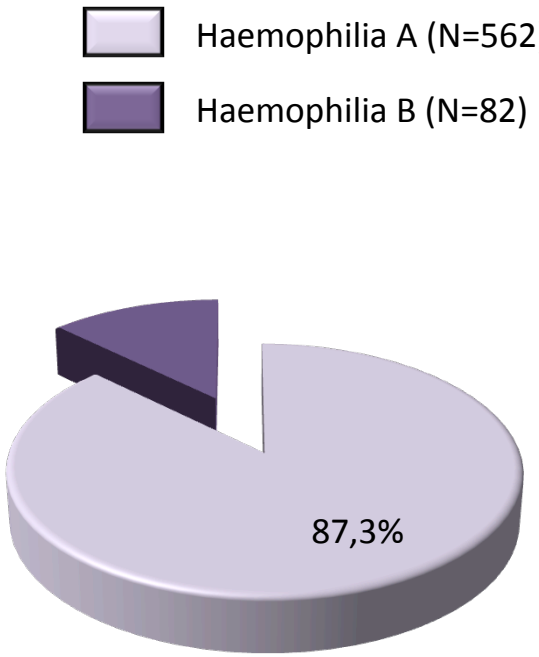


** Missing information on year of diagnosis in 167 persons.*

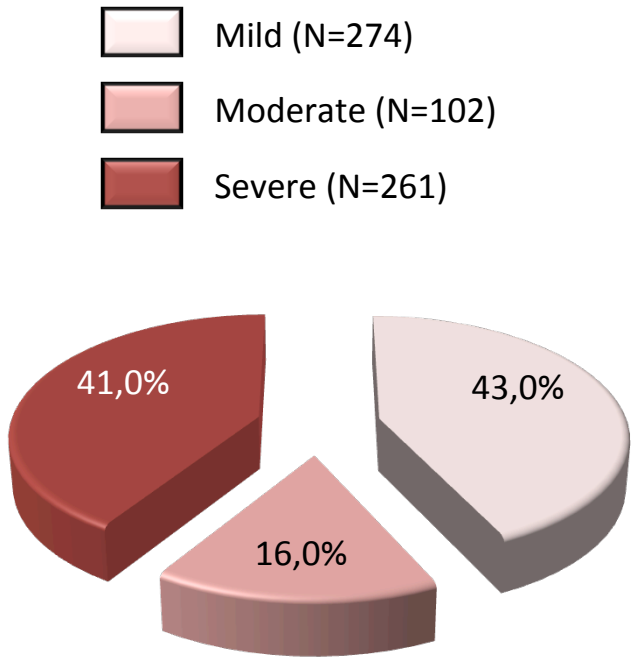
Type and severity of haemophilia I

All
N=644

Type of haemophilia



Severity of haemophilia (N=637*)

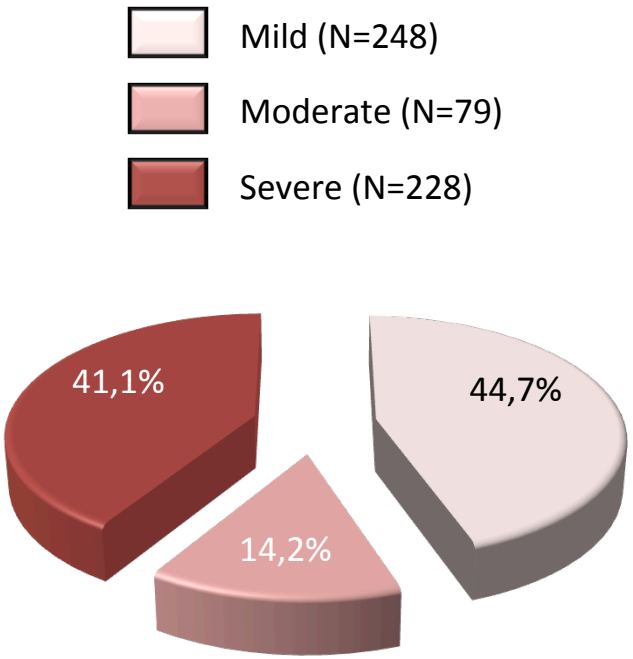


* *Severity of haemophilia not known in 7 persons.*

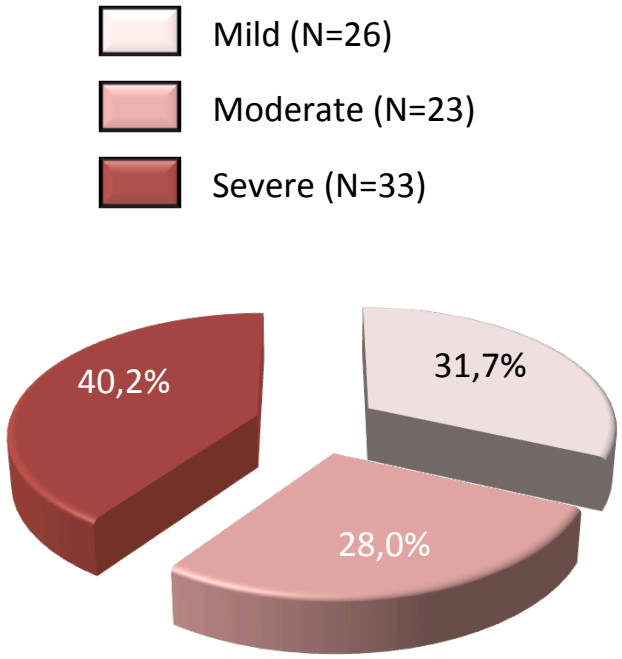
Type and severity of haemophilia II

All
N=644

Haemophilia A (N=555[1)])



Haemophilia B (N=82)

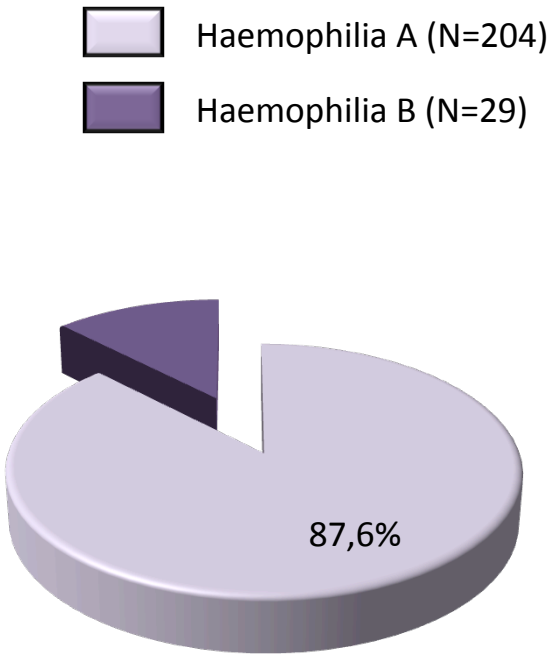


[1)] *Severity not known in 7 persons with haemophilia A.*

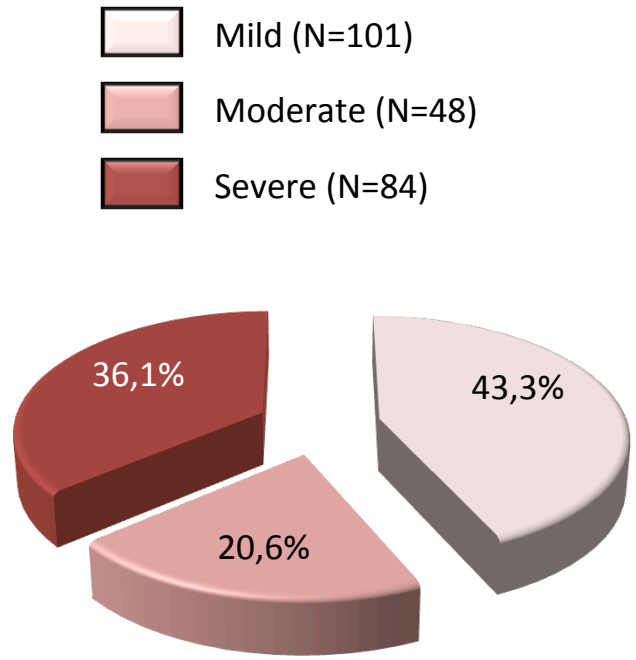
Type and severity of haemophilia I

Children
N=233

Type of haemophilia



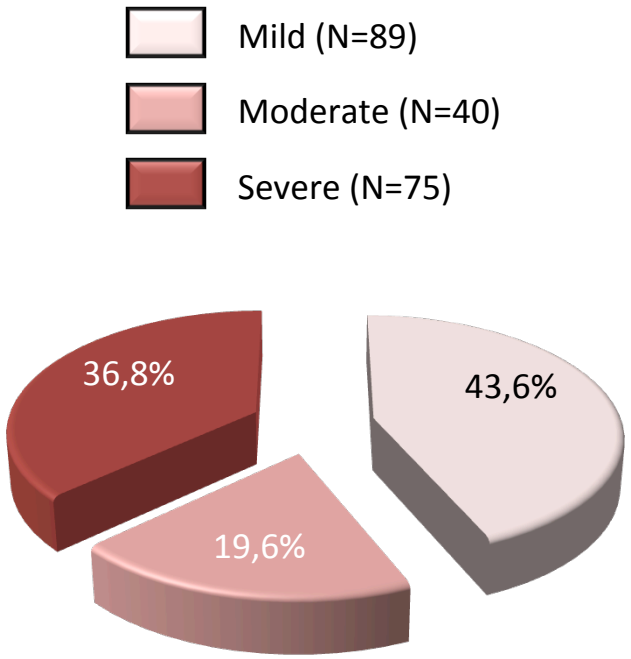
Severity of haemophilia (N=216)



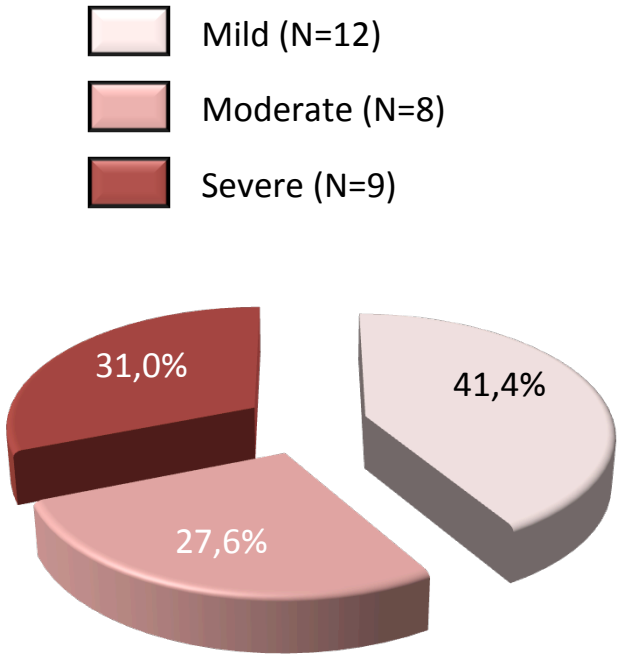
Type and severity of haemophilia II

Children
N=233

Haemophilia A (N=204)



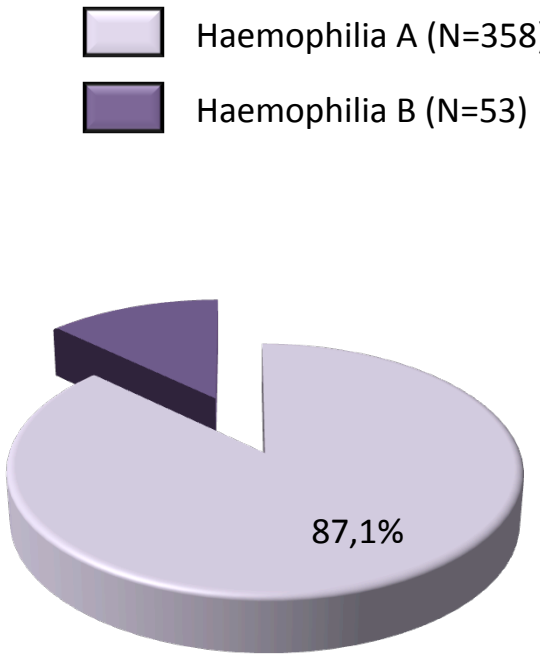
Haemophilia B (N=29)



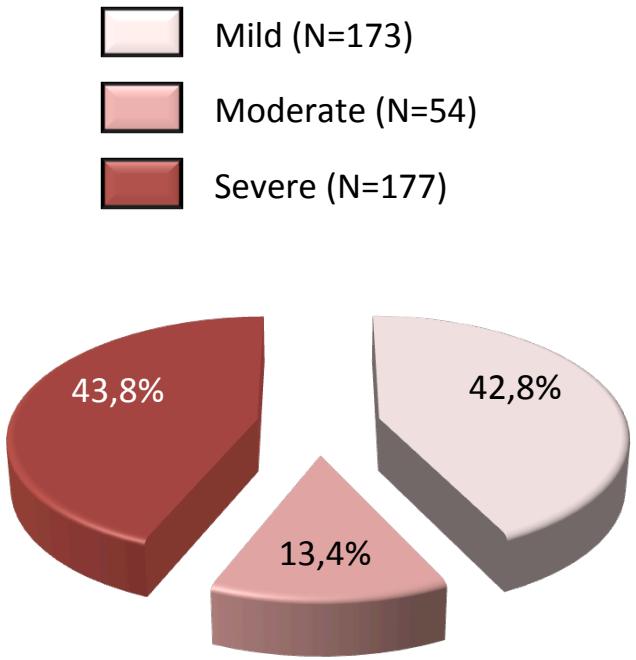
Type and severity of haemophilia I

Adults
N=411

Type of haemophilia



Severity of haemophilia (N=404*)

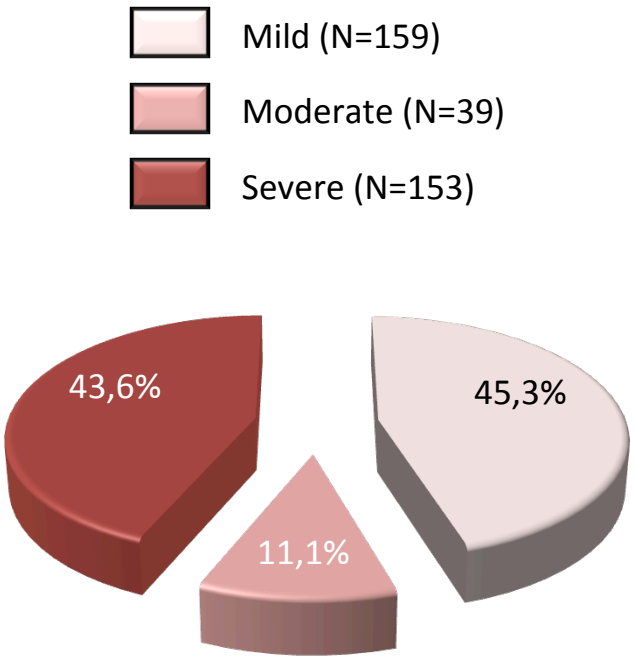


* *Severity of haemophilia not known in 7 adults.*

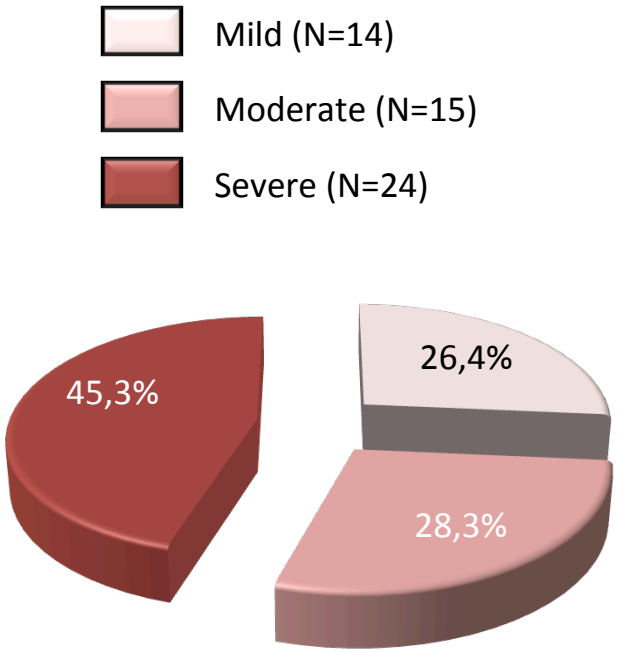
Type and severity of haemophilia II

Adults
N=411

Haemophilia A (N=351[1)])



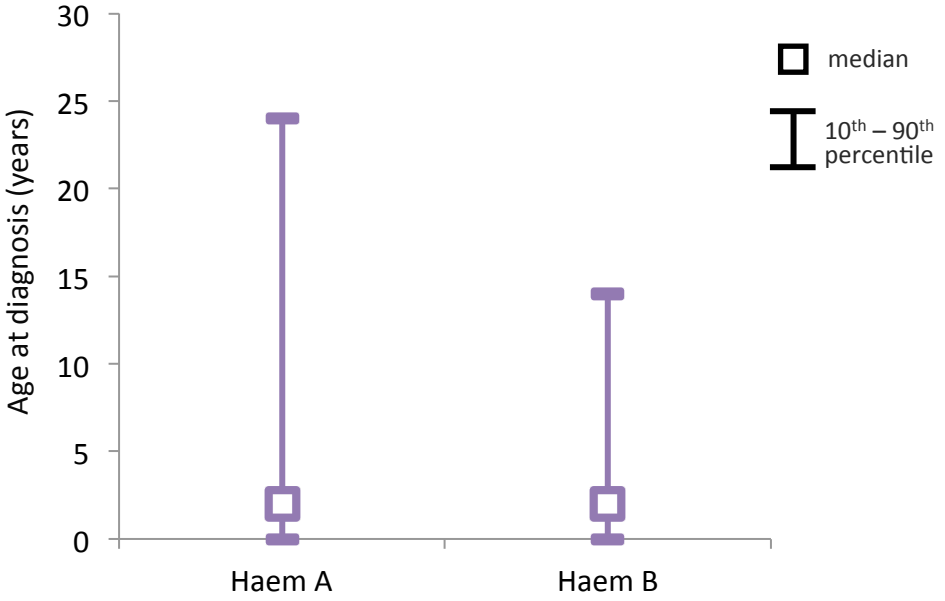
Haemophilia B (N=53)

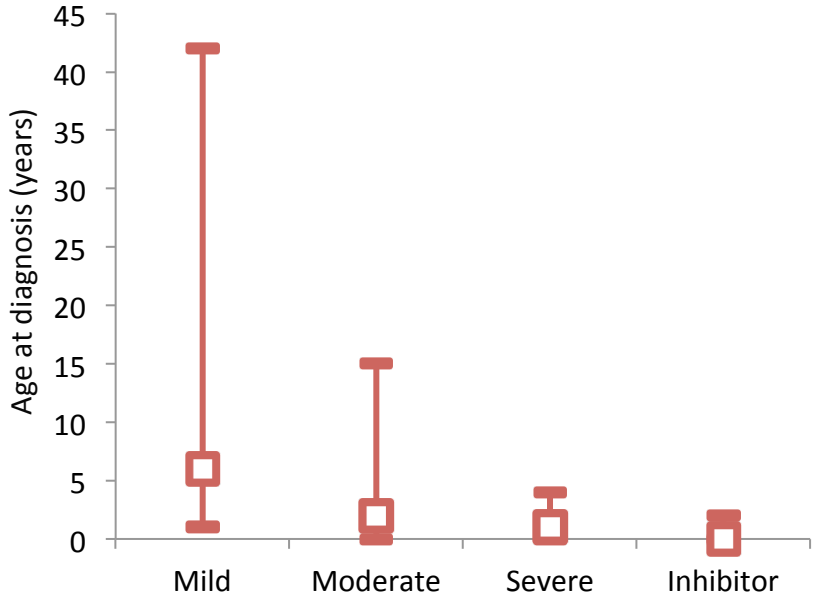


[1)] *Severity not known in 7 adults with haemophilia A.*

Age at diagnosis according to type and severity of haemophilia

All
N=644



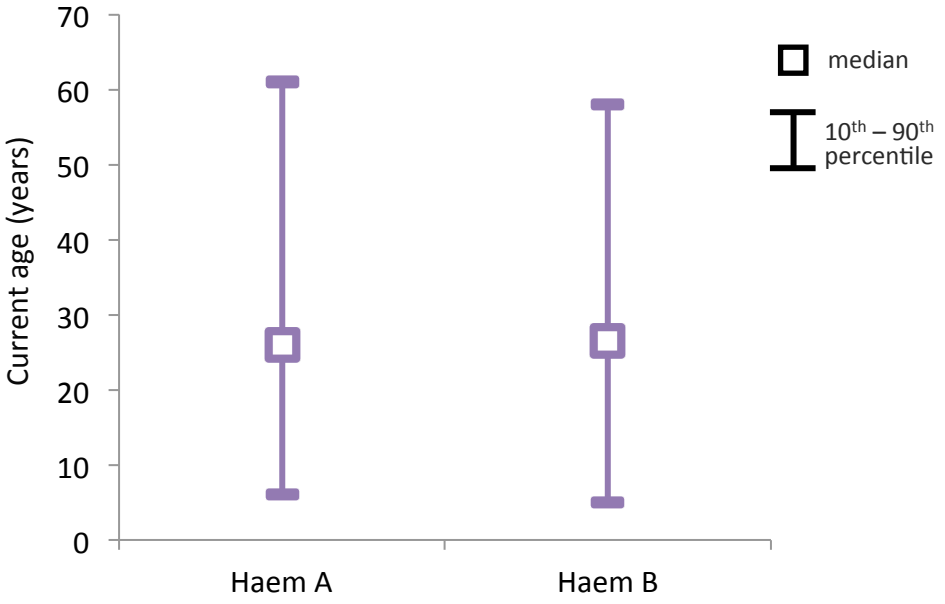


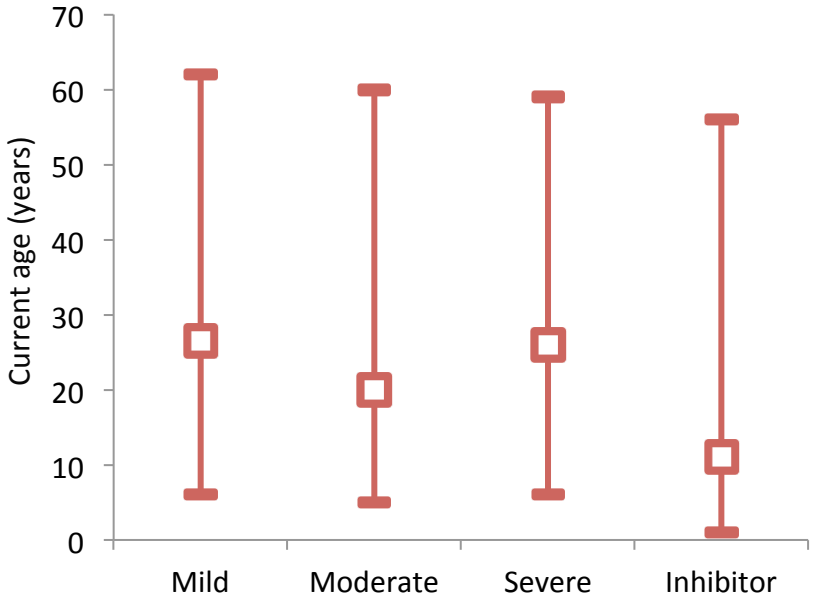
Haemophilia A	Haemophilia B	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor
562	*82*	**N**	*274*	*102*	*261*	*9*
7.7	*6.9*	**Mean**	*13.3*	*5.2*	*2.0*	*0.8*
2 (0 – 69)	*2 (0 – 63)*	**Median (min – max)**	*6 (0 – 69)*	*2 (0 – 61)*	***1*** *(0 – 48)*	*0 (0 – 2)*

** including persons with inhibitor*

Current age according to type and severity of haemophilia

All
N=644





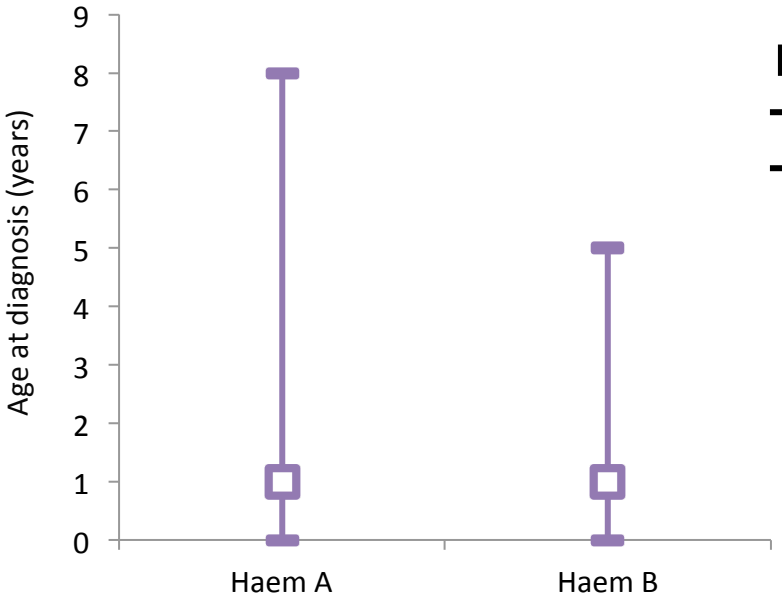
Haemophilia A	Haemophilia B	Current age† (years)	Mild*	Moderate*	Severe*	Inhibitor
562	*82*	**N**	*274*	*102*	*261*	*9*
30.5	*30.2*	**Mean**	*31.5*	*27.4*	*29.9*	*19.9*
26 (0 – 90)	*26.5 (2 – 67)*	**Median (min – max)**	*26.5 (0 – 90)*	*20 (0 – 71)*	*26 (0 – 73)*	*11 (1 – 56)*

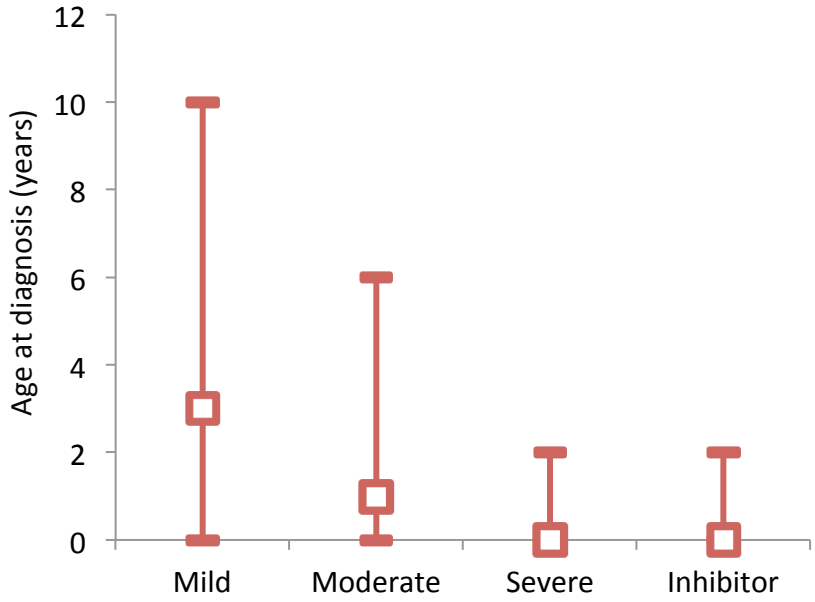
†*Current age = age reached in year 2012*

* *including persons with inhibitor*

Age at diagnosis according to type and severity of haemophilia

Children
N=233



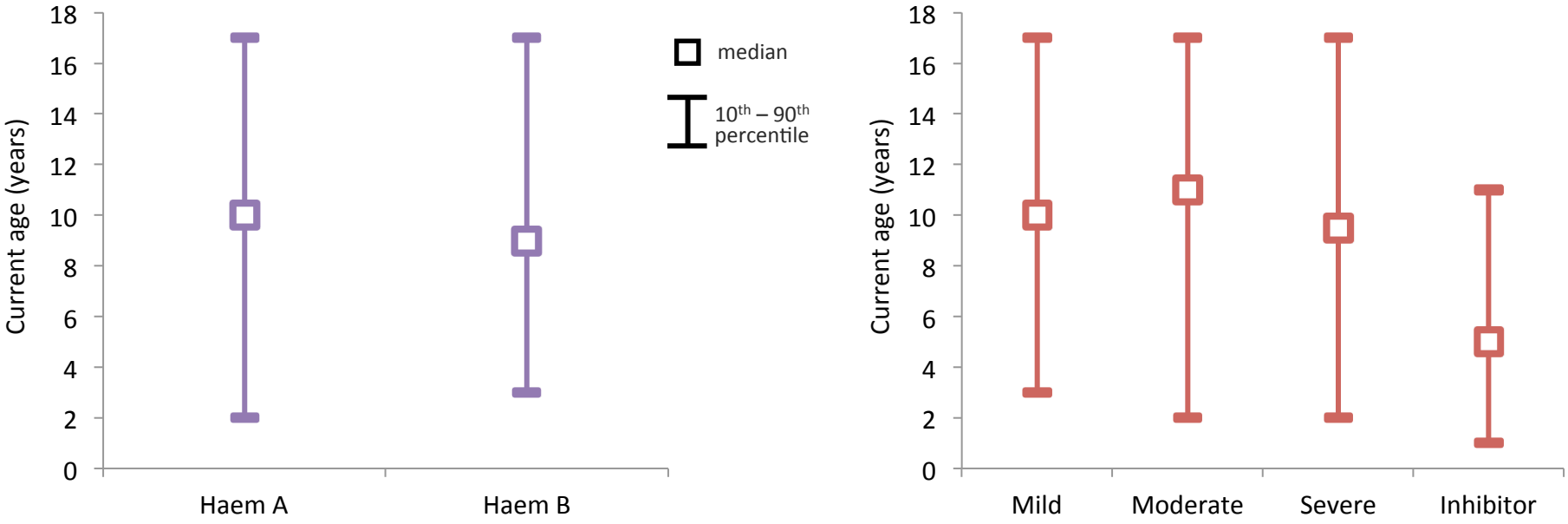


Haemophilia A	Haemophilia B	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor
204	*29*	**N**	*101*	*48*	*84*	*5*
2.6	*2.3*	**Mean**	*4.0*	*2.6*	***0.9***	*0*
1 (0 – 17)	*1 (0 –13)*	**Median (min – max)**	*3 (0 – 13)*	*1 (0 – 17)*	***0*** *(0 – 11)*	*0 (0 – 2)*

** including persons with inhibitor*

Actual age according to type and severity of haemophilia

Children
N=233



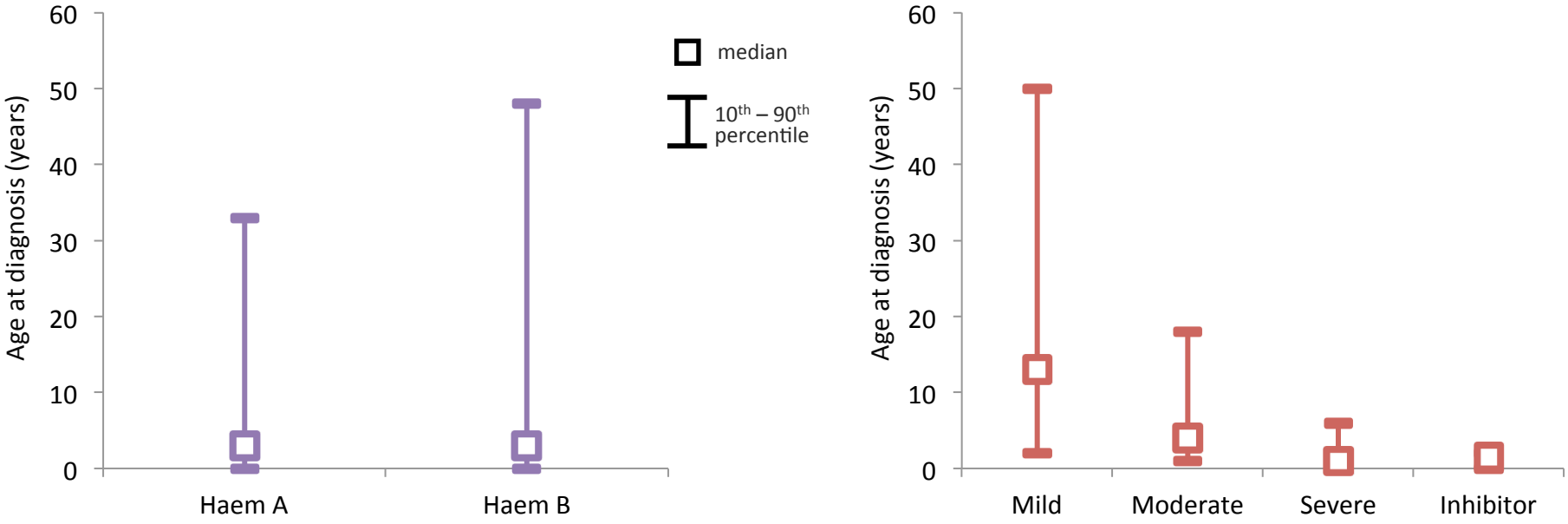
Haemophilia A	Haemophilia B	Current age* (years)	Mild*	Moderate*	Severe*	Inhibitor
204	*29*	**N**	*101*	*48*	*84*	*5*
9.8	*9.6*	**Mean**	***9.8***	***10.5***	***9.3***	***5.4***
10 (0 – 18)	*9 (2 – 18)*	**Median (min – max)**	*10 (0 – 18)*	*11 (0 – 18)*	*9.5 (0 – 18)*	*5 (1 – 11)*

**Current age = age reached in year 2012*

** including persons with inhibitor*

Age at diagnosis according to type and severity of haemophilia

Adults
N=411

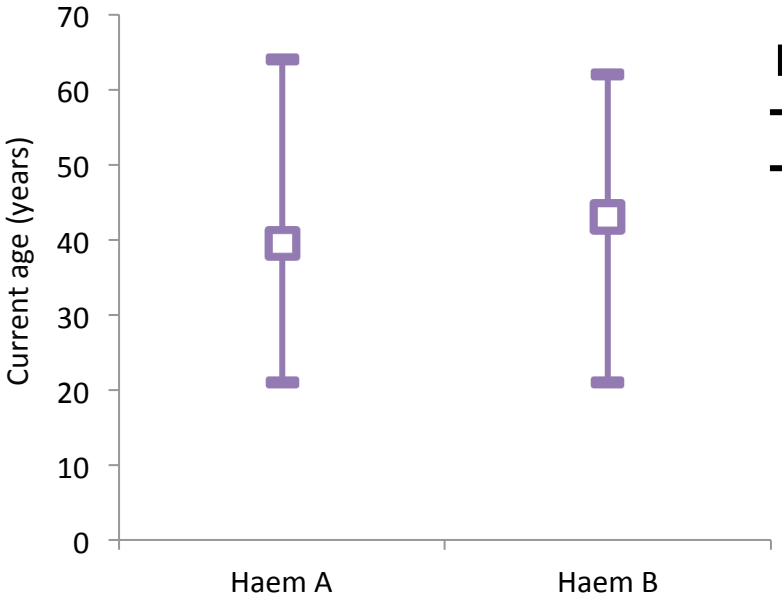


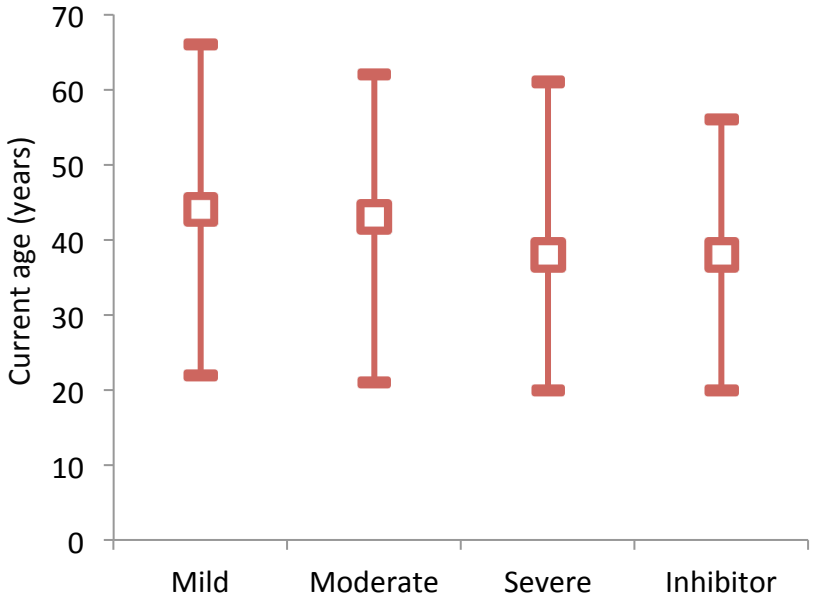
Haemophilia A	Haemophilia B	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor
358	*53*	**N**	*173*	*54*	*177*	*4*
10.9	*10.6*	**Mean**	*19.3*	*7.4*	*2.6*	*1.3*
3 (0 – 69)	*3 (0 – 63)*	**Median (min – max)**	*13 (1 – 69)*	*4 (0 – 61)*	*1 (0 – 48)*	*1.5 (0 – 2)*

** including persons with inhibitor*

Actual age according to type and severity of haemophilia

Adults
N=411





Haemophilia A	Haemophilia B	Current age* (years)	Mild*	Moderate*	Severe*	Inhibitor
358	*53*	**N**	*173*	*54*	*177*	*4*
42.3	*41.5*	**Mean**	***44.2***	***42.5***	***39.7***	***38.0***
39.5 (19 – 90)	*43 (20 – 67)*	**Median (min – max)**	*44 (19 – 90)*	*43 (19 – 71)*	*38 (19 – 73)*	*38 (20 – 56)*

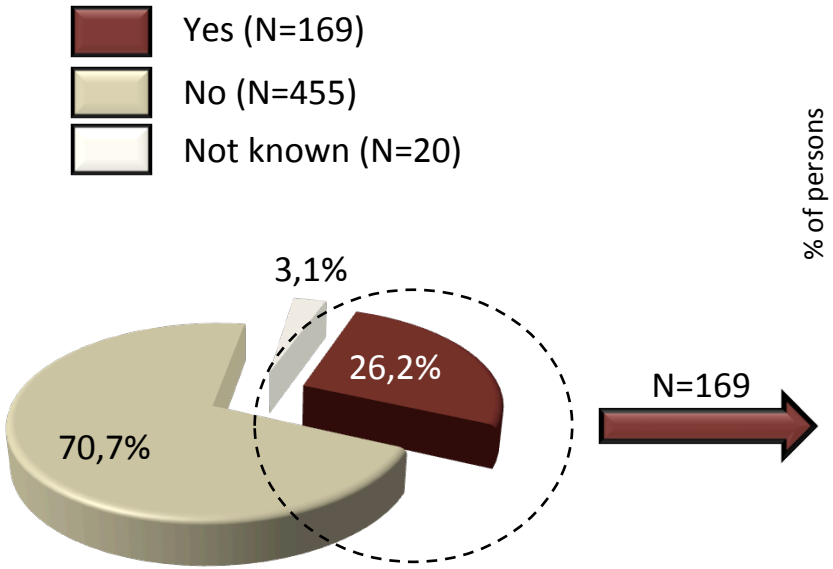
**Current age = age reached in year 2012*

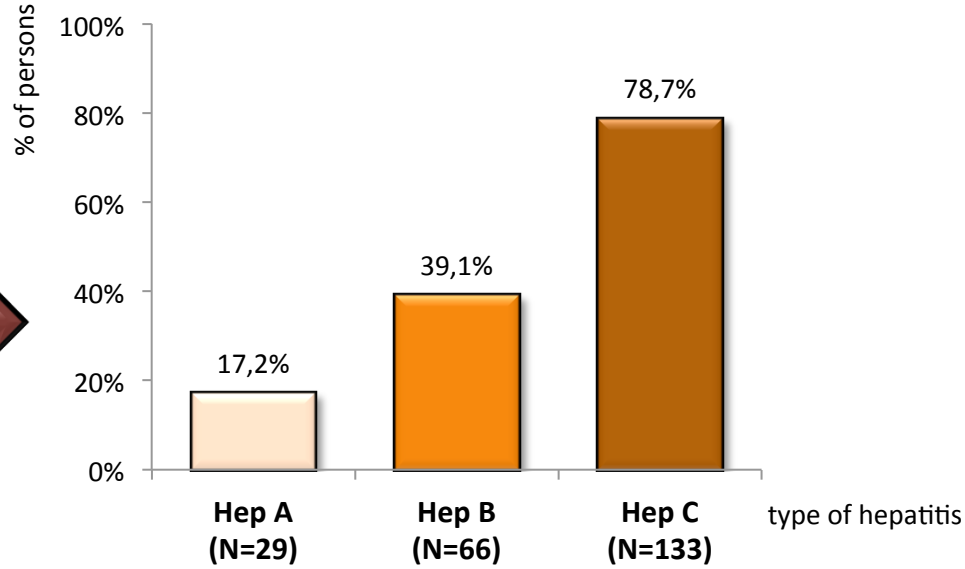
** including persons with inhibitor*

All
N=644

Hepatitis experienced

Experienced hepatitis





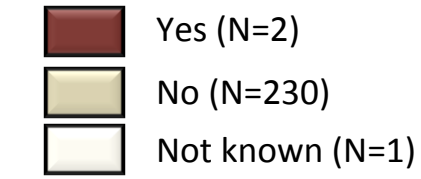
Data from last annual report of each person.

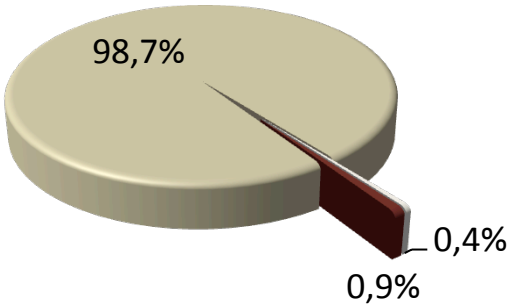
Type of hepatitis not specified in 5 persons. One person may have recorded more types of hepatitis.

Hepatitis experienced

Children
N=233

Experienced hepatitis





N=2

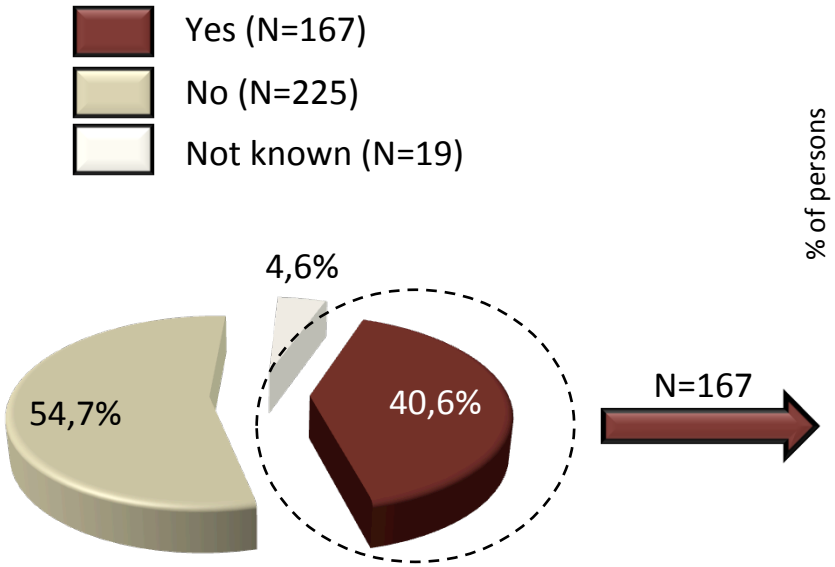
One child has hepatitis C, type of hepatitis not specified in one child.

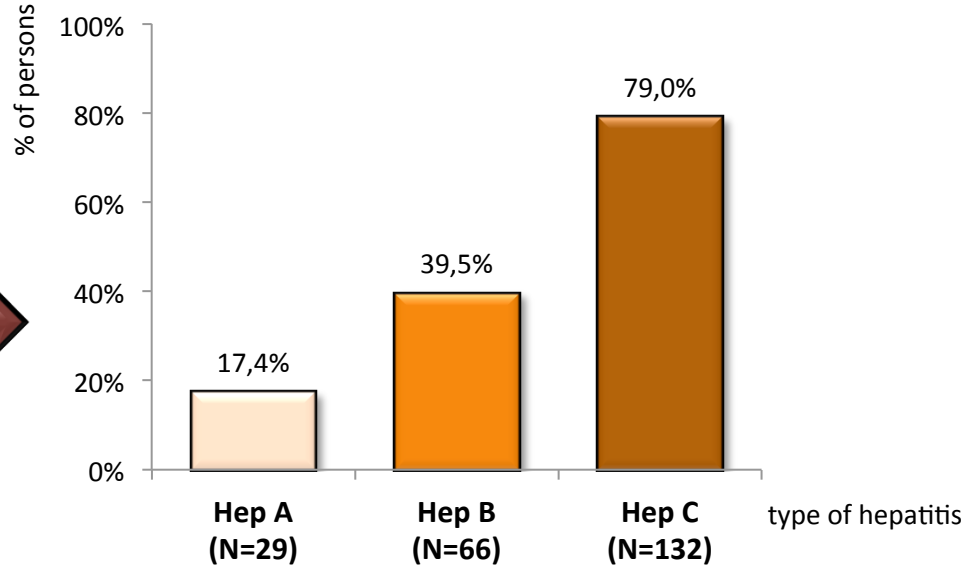
Data from last annual report of each person.

Hepatitis experienced

Adults
N=411

Experienced hepatitis





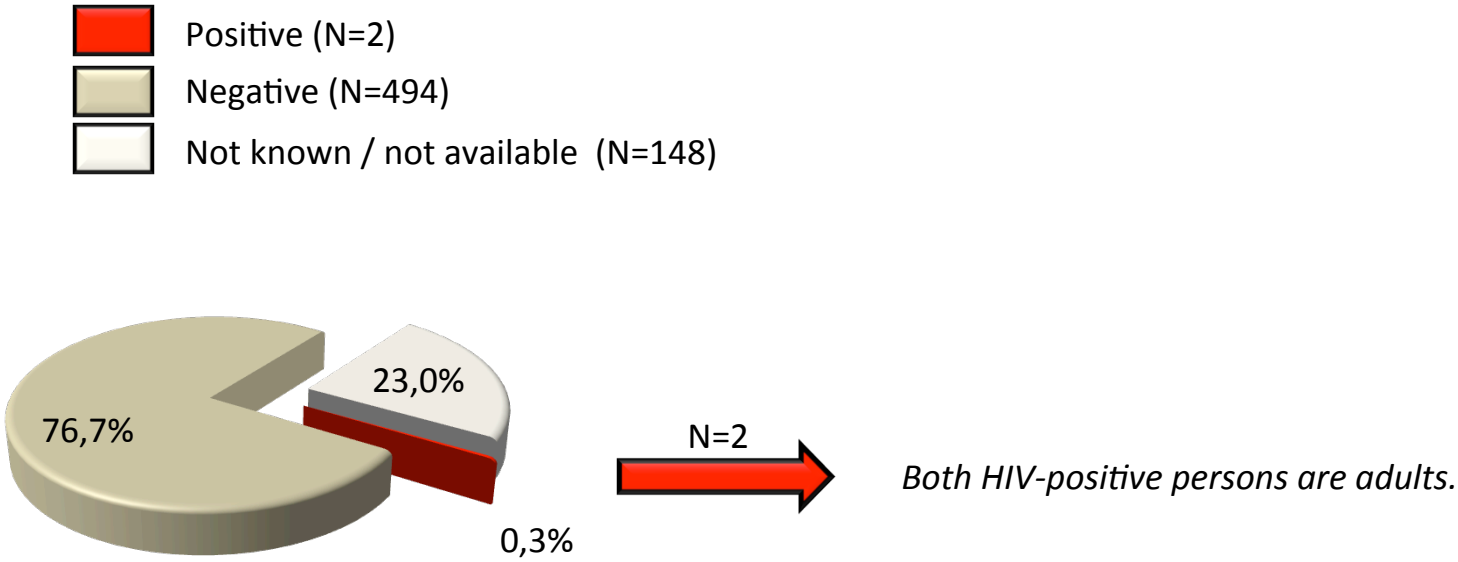
Data from last annual report of each person.

Type of hepatitis not specified in 3 adults. One person may have recorded more types of hepatitis.

All
N=644

HIV

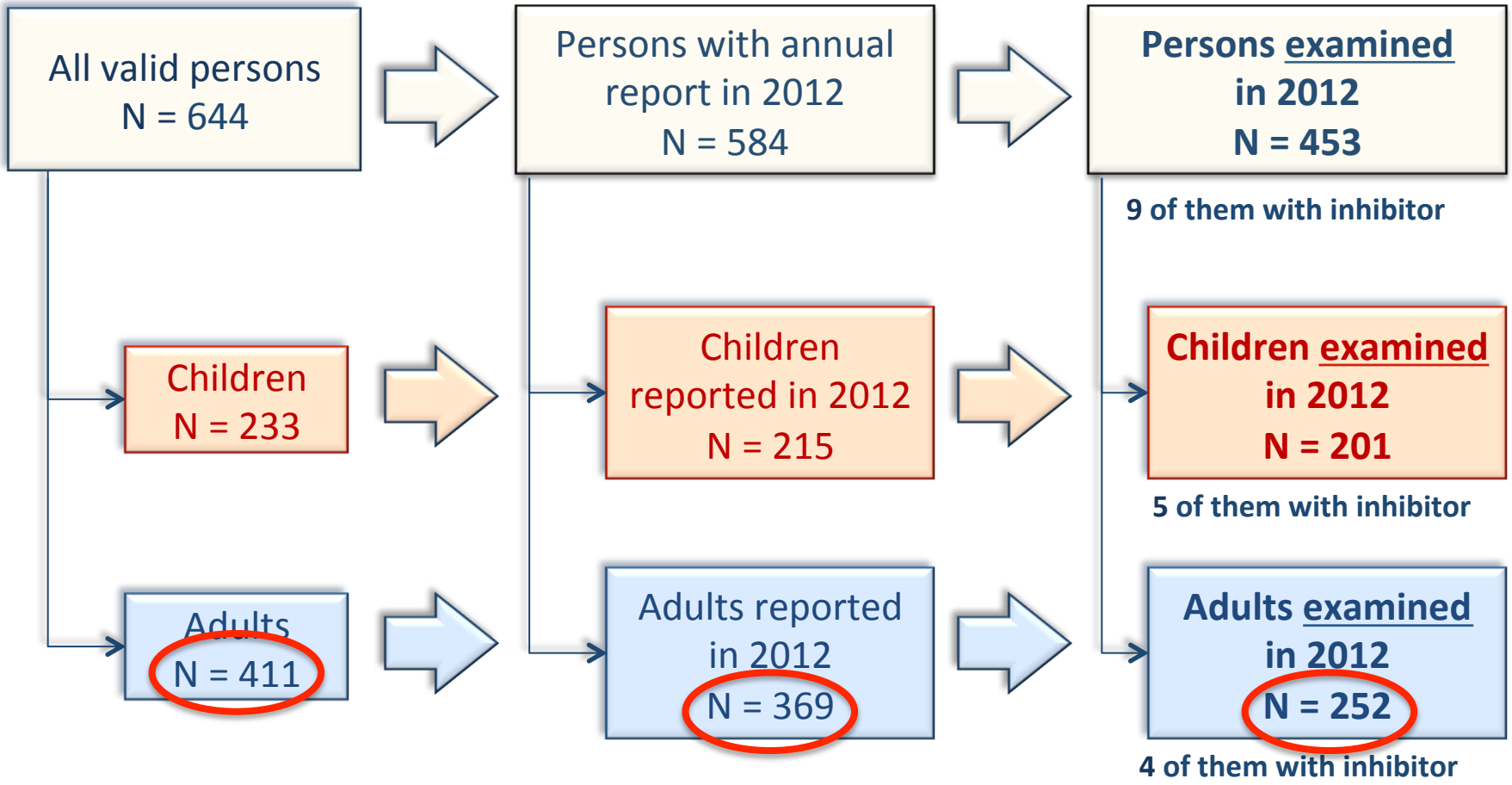
HIV



Both HIV-positive persons are adults.

Data from last annual report of each person.

Data from year 2012 – sample size



All
N=453

Persons with haemophilia with inhibitor

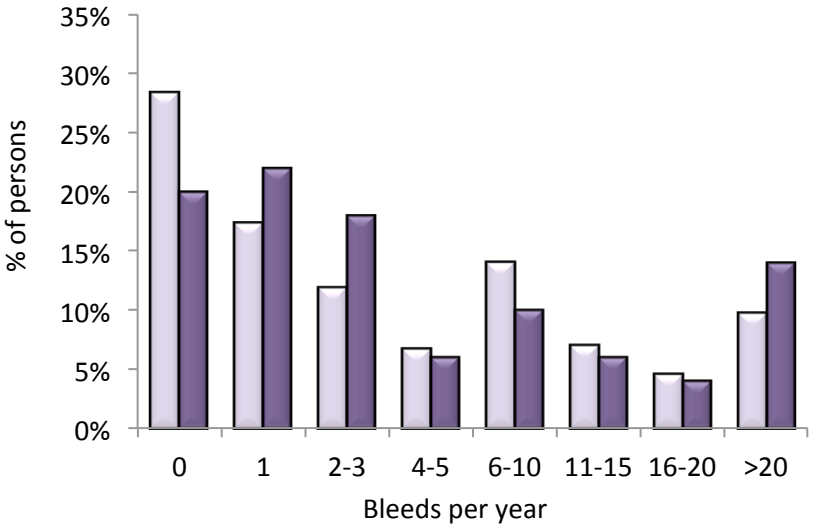
- inhibitor was recorded in 8 persons in year 2012
- other 1 person has recorded inhibitor in 2011 (data from 2012 are not available)

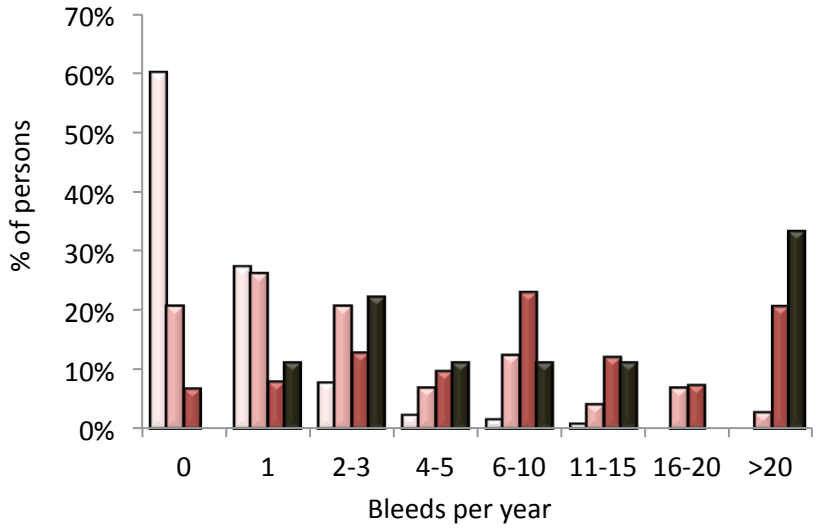
currently 9 persons with inhibitor

(5 children and 4 adults) + 6 in other centre

All
N=453

Frequency of bleeding requiring treatment in 2012





Haemophilia A	Haemophilia B	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
386	*67*	**N total**	*143*	*83*	*214*	*9*
327	*50*	**N valid**	*128*	*73*	*165*	*9*
7.2	*9.6*	**Mean**	*0.8*	*4.6*	*13.8 / **6.2**†*	*12.2*
2 (0 – 144)	*2 (0 – 132)*	**Median (min – max)**	*0 (0 – 12)*	*2 (0 – 31)*	*7 / **5** (0 – 144)*	*8 (1 – 32)*

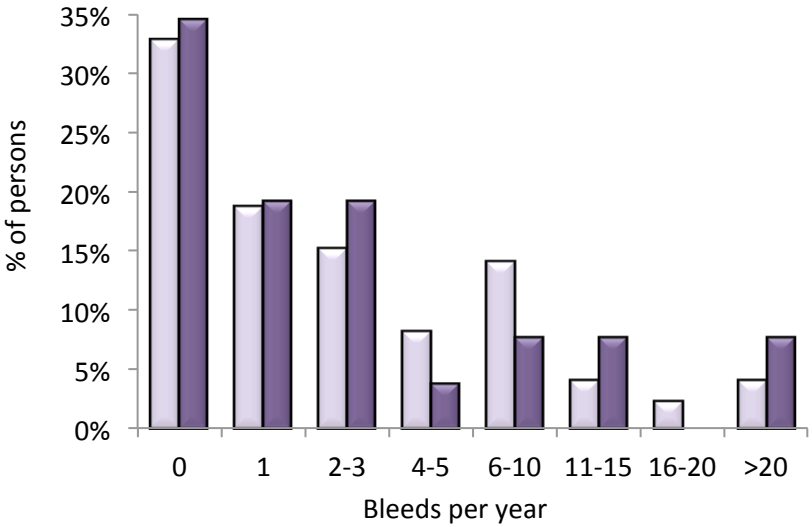
Frequency of bleeding is missing in 207 persons.

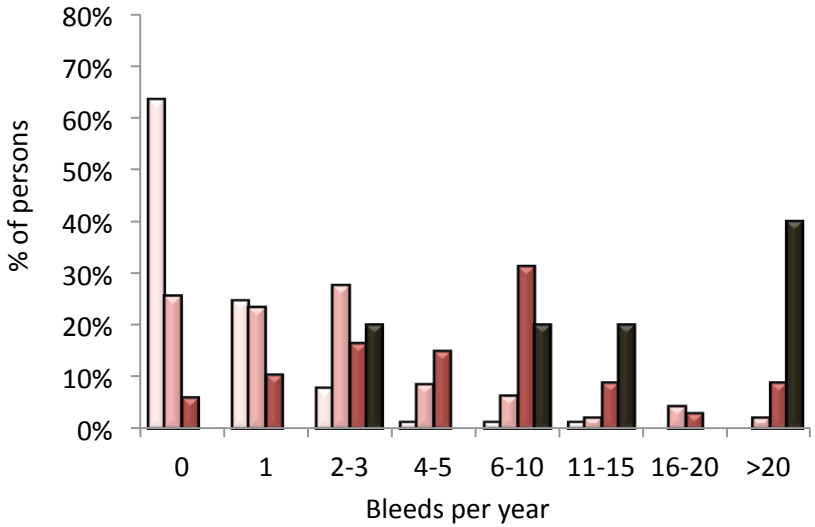
** without inhibitor*

† mean and median of frequency of bleeding in persons with severe haemophilia without inhibitor on permanent prophylaxis

Frequency of bleeding requiring treatment in 2012

Children
N=201





Haemophilia A	Haemophilia B	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
175	*26*	**N total**	*79*	*47*	*70*	*5*
170	*26*	**N valid**	*77*	*47*	*67*	*5*
4.3	*4.4*	**Mean**	*0.8*	*3.6*	*8.0 / **6.1**†*	*15.2*
1 (0 – 49)	*1 (0 – 26)*	**Median (min – max)**	*0 (0 – 12)*	*2 (0 – 30)*	*6 / **5** (0 – 49)*	*11 (3 – 32)*

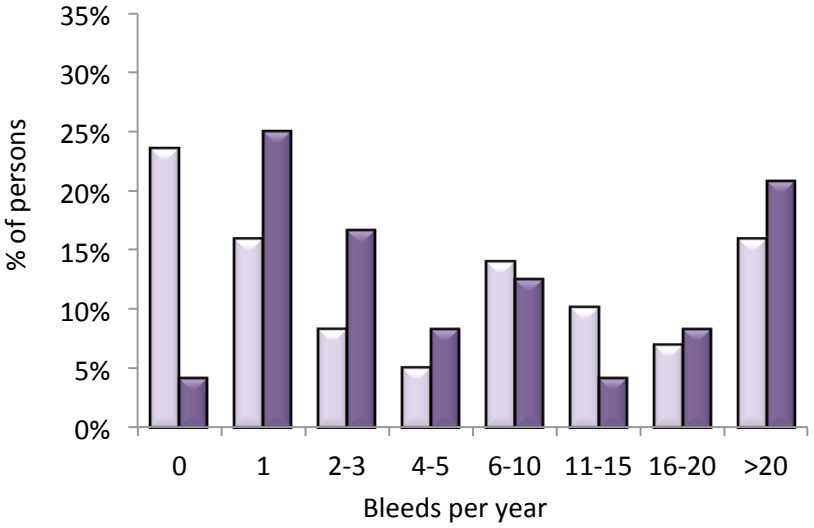
Frequency of bleeding is missing in 19 children.

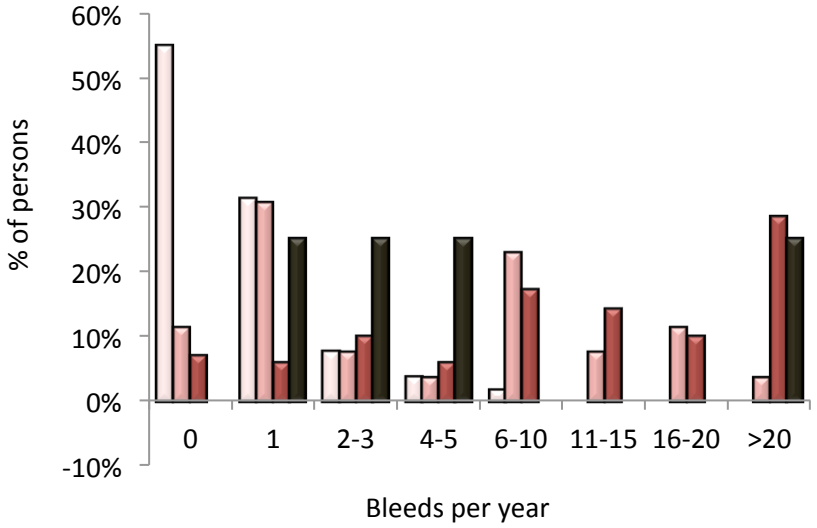
** without inhibitor*

† mean and median of frequency of bleeding in children with severe haemophilia without inhibitor on permanent prophylaxis

Frequency of bleeding requiring treatment in 2012

Adults
N=252





Haemophilia A	Haemophilia B	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
211	*41*	**N total**	*64*	*36*	*144*	*4*
157	*24*	**N valid**	*51*	*26*	*98*	*4*
10.5	*15.3*	**Mean**	*0.8*	*6.5*	*17.7 / **6.3**†*	*8.5*
5 (0 – 144)	*5 (0 – 132)*	**Median (min – max)**	*0 (0 – 7)*	*3.5 (0 – 31)*	*12.5/**4** (0 – 144)*	*3.5 (1 – 26)*

Frequency of bleeding is missing in 188 adults.

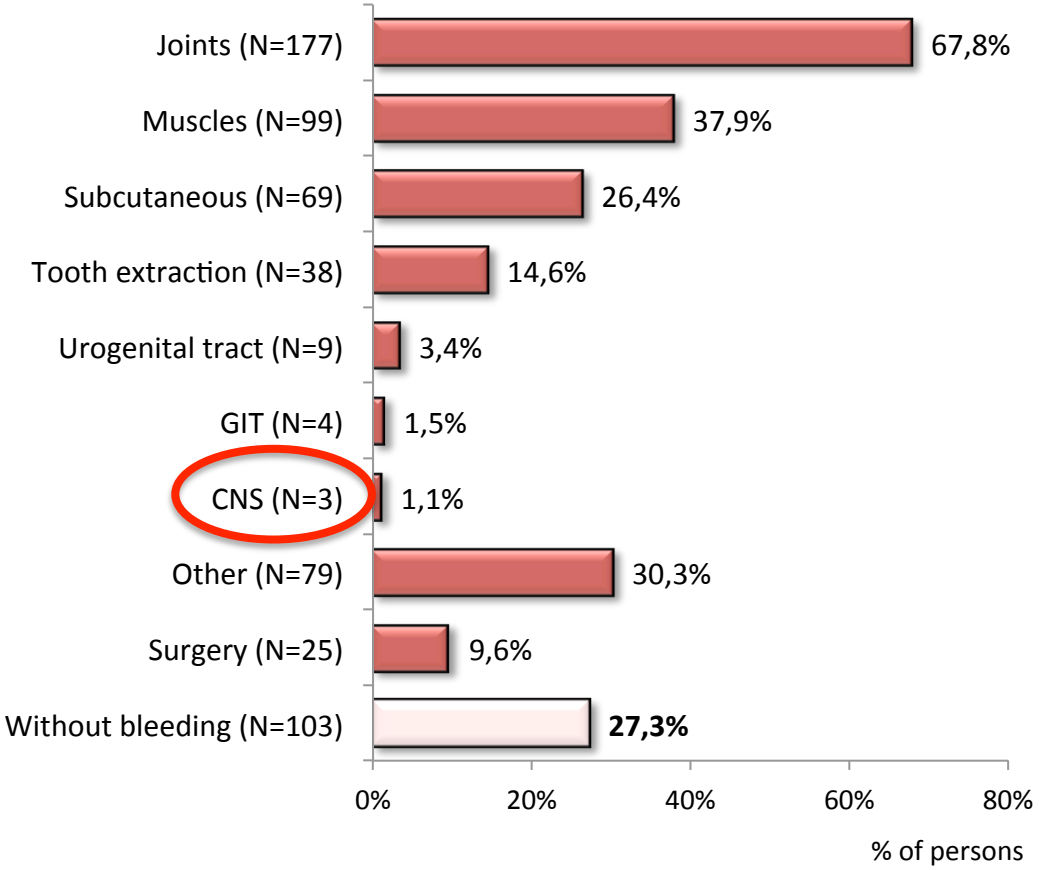
** without inhibitor*

† mean and median of frequency of bleeding in adults with severe haemophilia without inhibitor on permanent prophylaxis

Location of (any) bleeds in 2012

All
N=453

274 (72.7%) persons experienced bleeding requiring treatment at least once in year; 2849 bleeds were recorded in total, 78 bleeds required hospitalization.
103 (27.3%) persons recorded no bleed during year 2012.
Information on frequency of bleeding is missing in 76 examined persons.
Overall 261 persons have recorded location of any bleeds*.

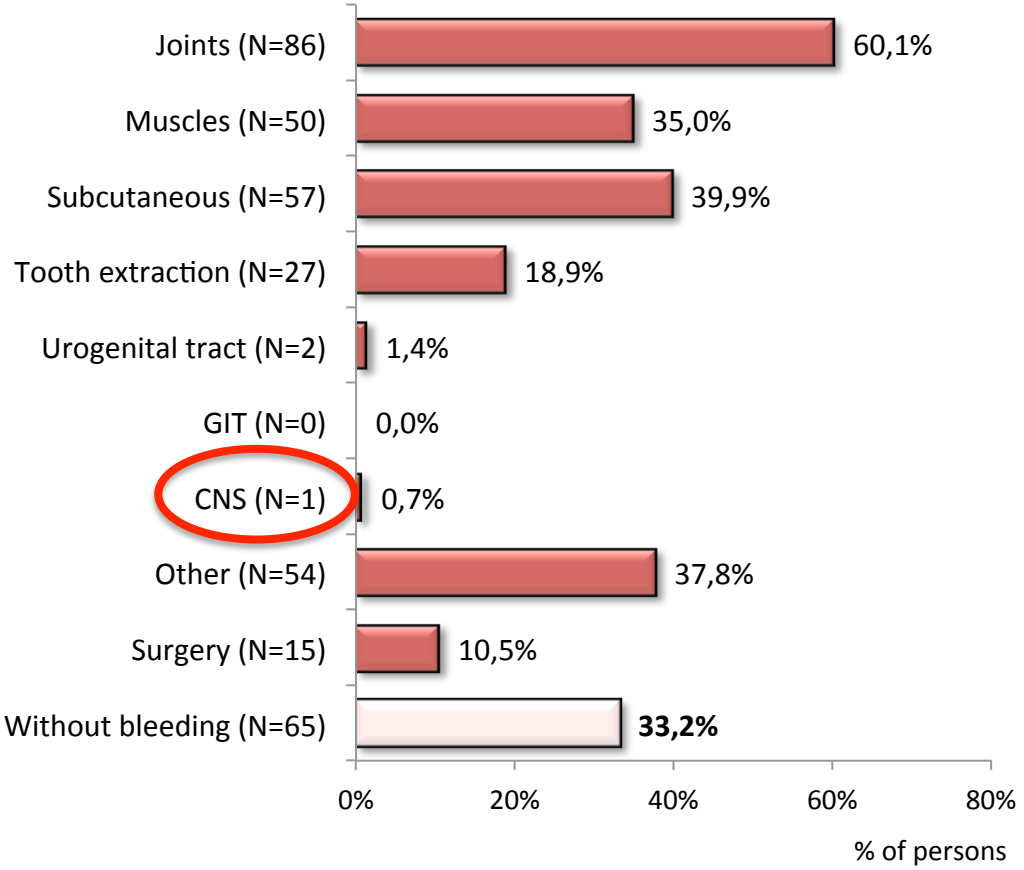


** all recorded bleeds, regardless of requirement of treatment*

Location of (any) bleeds in 2012

Children
N=201

131 (66.8%) children experienced bleeding requiring treatment at least once in year; 840 bleeds were recorded in total, 50 bleeds required hospitalization.
65 (33.2%) children recorded no bleed during year 2012.
Information on frequency of bleeding is missing in 5 examined children.
Overall 143 persons have recorded location of any bleeds*.

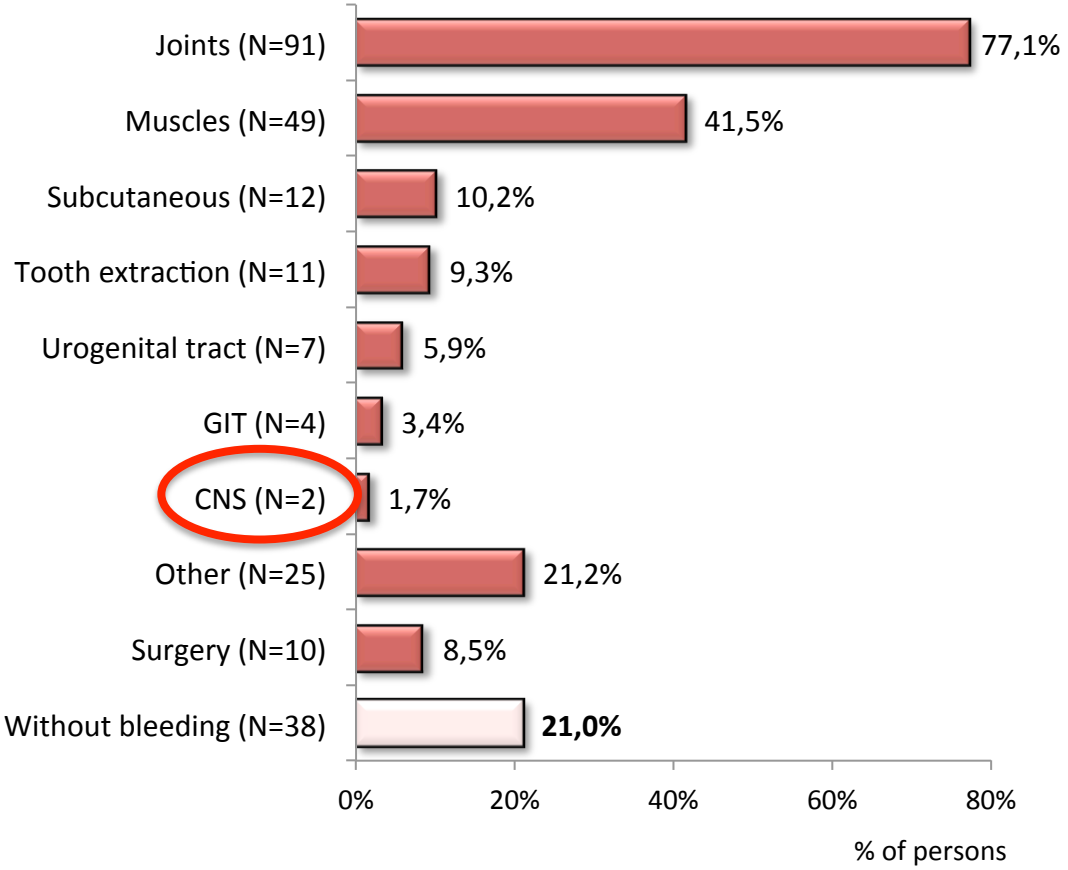


** all recorded bleeds, regardless of requirement of treatment*

Location of (any) bleeds in 2012

Adults
N=252

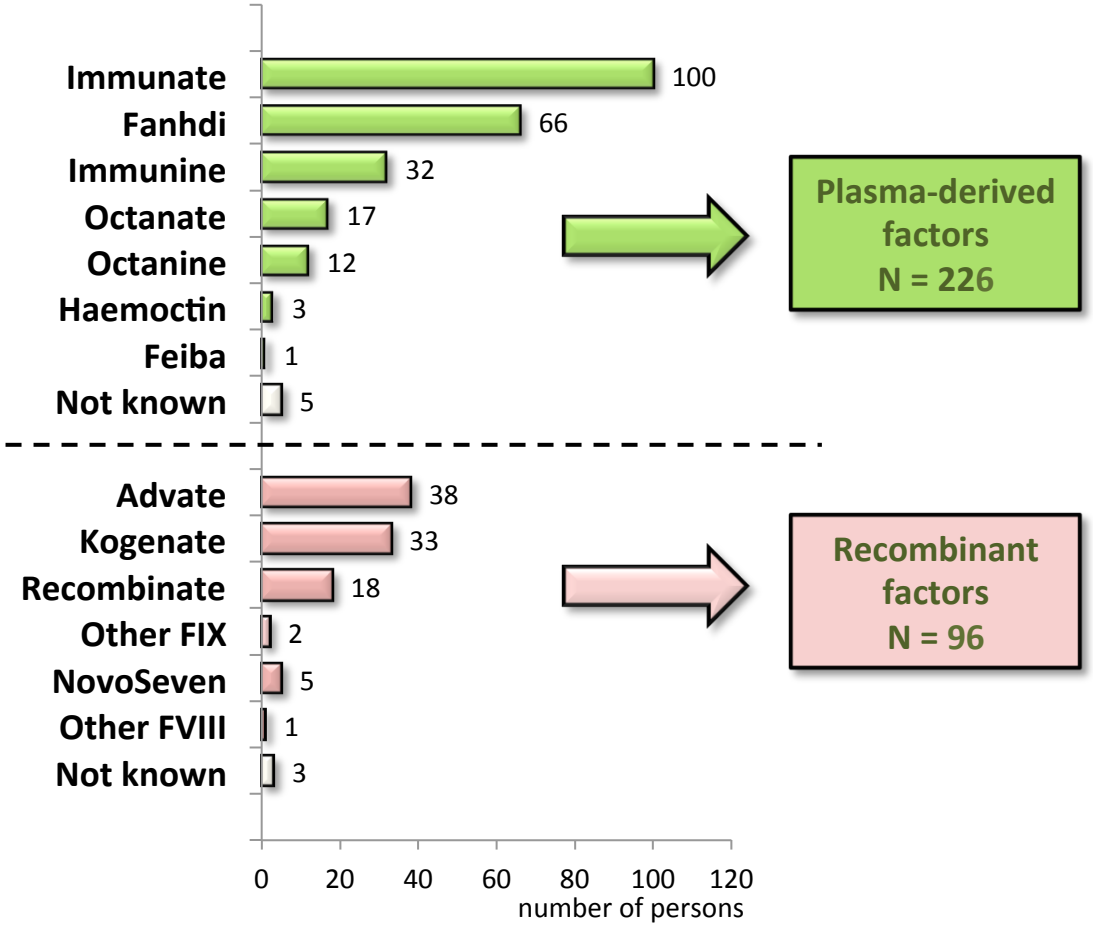
143 (79%) adults experienced bleeding requiring treatment at least once in year; 2009 bleeds were recorded in total, 28 bleeds required hospitalization.
38 (21%) adults have recorded no bleed during year 2012.
Information on frequency of bleeding is missing in 71 examined adults.
Overall 118 persons have recorded location of any bleeds*.



** all recorded bleeds, regardless of requirement of treatment*

All
N=453

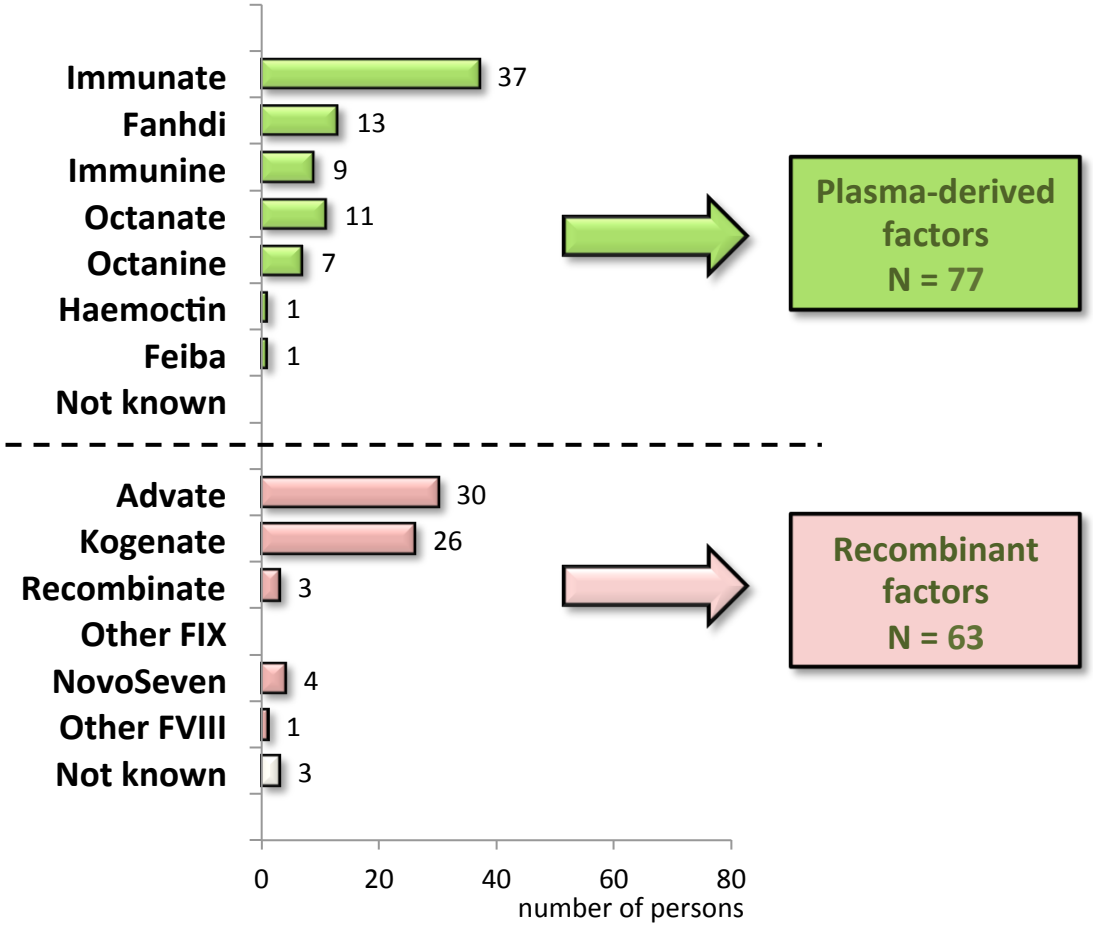
Treatment



312 (68.9%) persons received drugs in 2012 (20 of them more different drugs).
Plasma-derived factors were administered more frequently – in 226 (50%) persons, whereas recombinant factors in 96 (21.2%) persons.
Ten persons were treated with both plasma-derived and recombinant factor.

Children
N=201

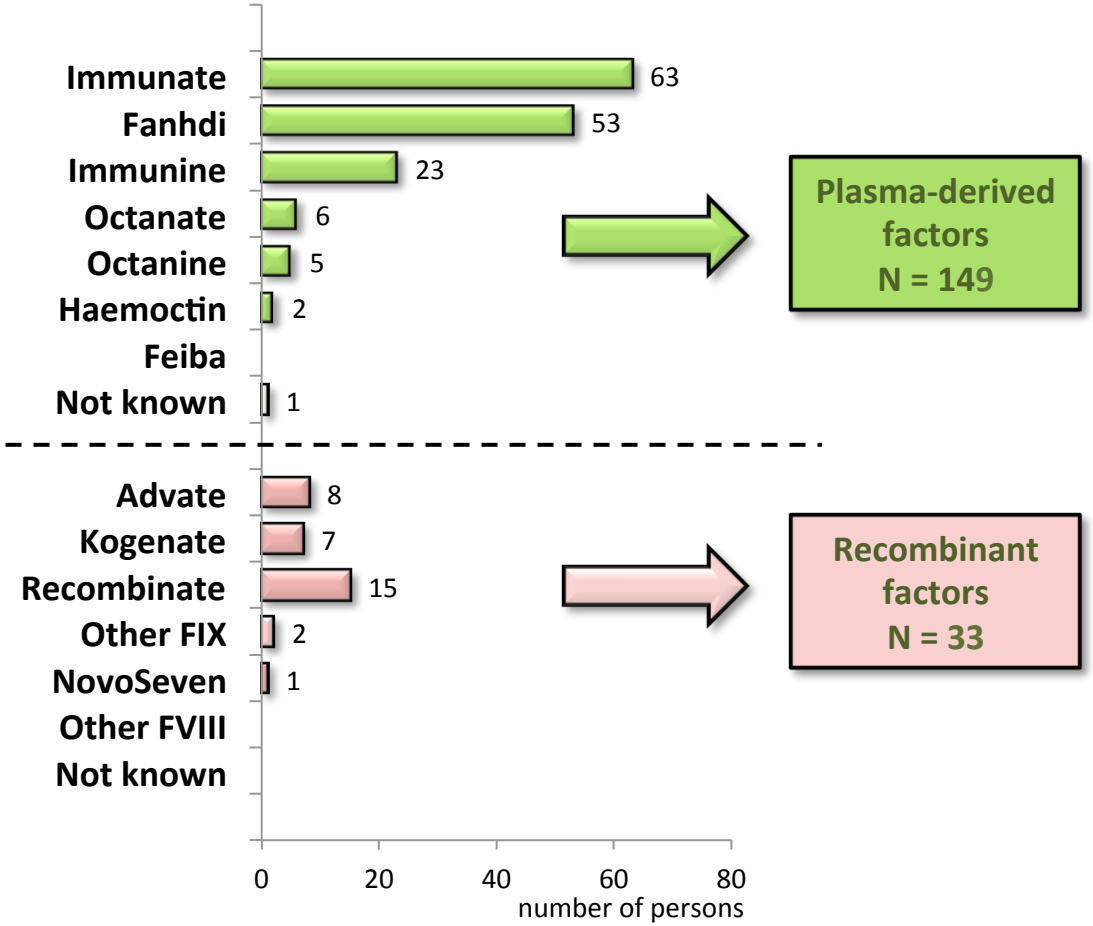
Treatment



135 (67.2%) children received drugs in 2012 (11 of them more different drugs).
Plasma-derived factors were administered in 77 (38.3%) children, recombinant factors in 63 (31.3%).
Five children were treated with both plasma-derived and recombinant factor.

Treatment

Adults
N=252



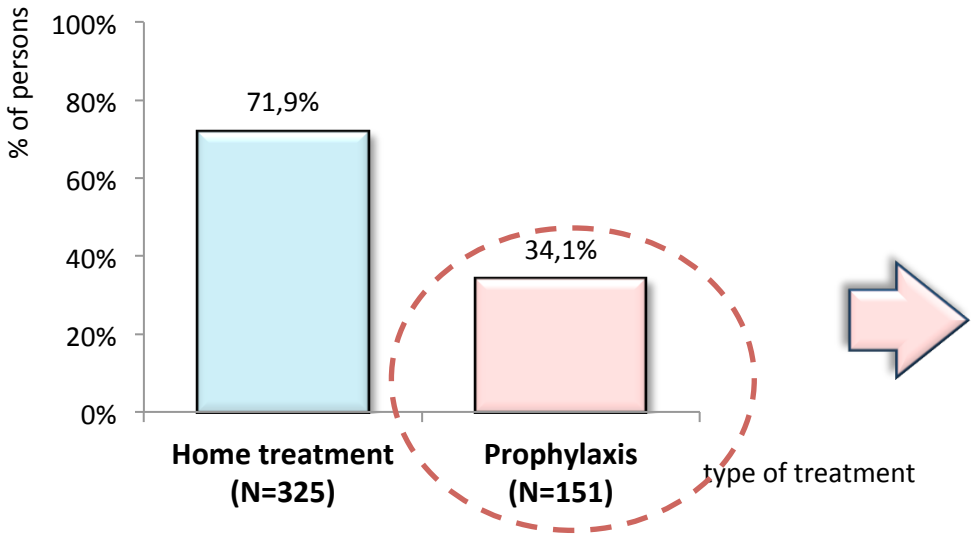
177 (70.2%) adults received drugs in 2012 (9 of them more different drugs).
Plasma-derived factors were administered more frequently – in 149 (59.1%) adults, whereas recombinant factors in 33 (13.1%) adults.
Five adults were treated with both plasma-derived and recombinant factor.

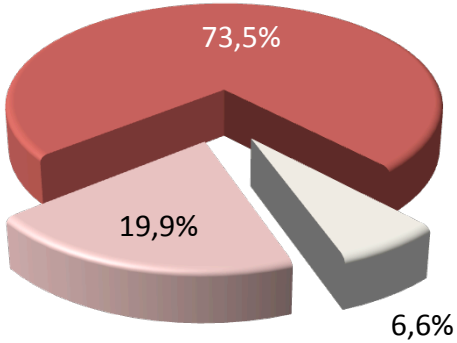
Comparison of treatment in years 2011 and 2012

		Treatment in 2012				
	Number of persons treated with:	Plasma-derived factor	Recombinant factor	All with treatment	Without treatment	Total
Treatment in 2011	Plasma-derived factor	**141**	**29**	164	63	227
		21.7%	**4.5%**	25.2%	9.7%	34.9%
	Recombinant factor	**3**	**44**	46	7	53
		0.5%	**6.8%**	7.1%	1.1%	8.1%
	All persons with treatment	144	57	194	70	264
		22.1%	8.8%	29.8%	10.8%	40.6%
	Without treatment	82	38	117	270	387
		12.6%	5.8%	18.0%	41.5%	59.4%
	Total	226	95	311	340	**651**
		34.7%	14.6%	47.8%	52.2%	**100.0%**

Type of treatment

All
N=453



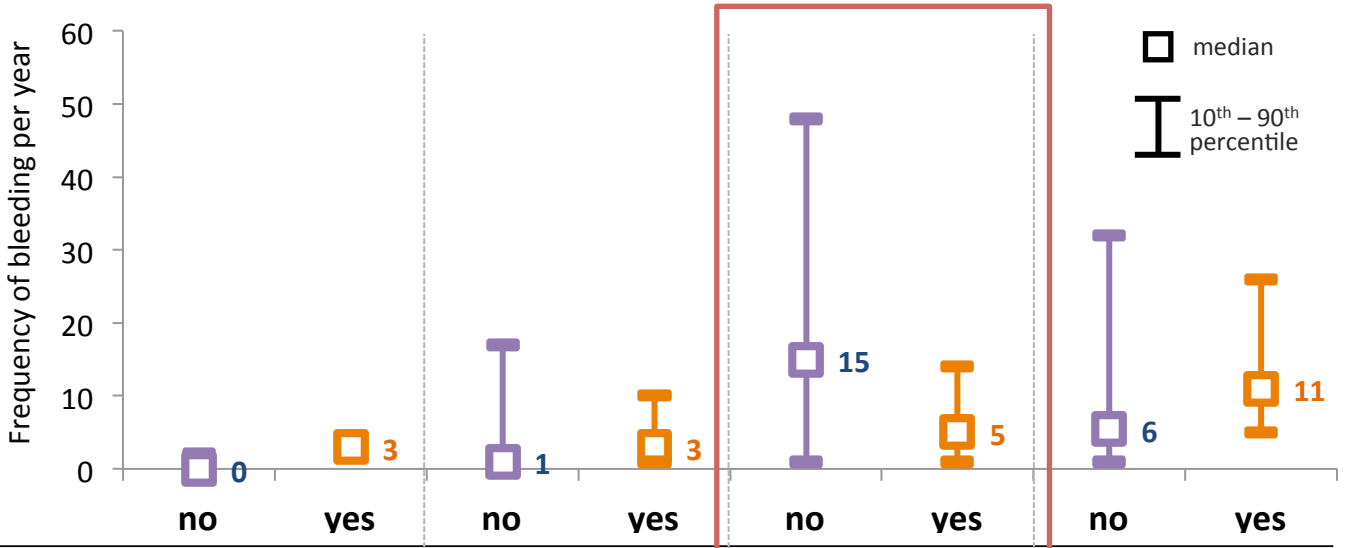


Type of prophylaxis (N=151)

- Temporary (N=30)
- Permanent (N=111)
- Not available (N=10)

Bleeding requiring treatment according to prophylaxis

All
N=453



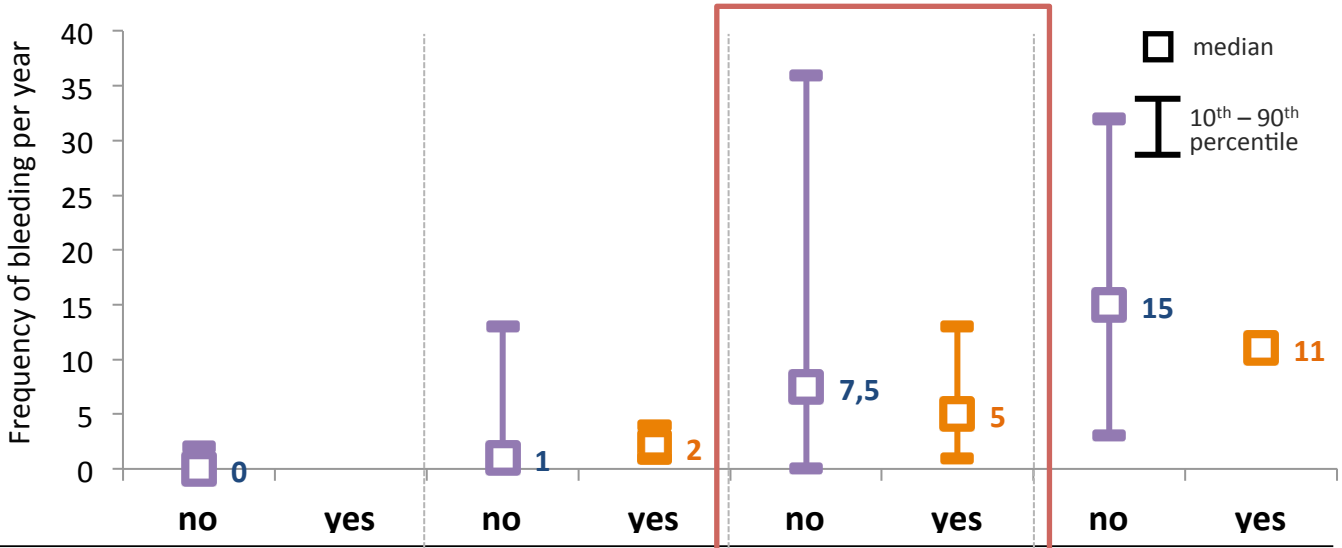
permanent prophylaxis

Frequency of bleeding	Mild*		Moderate*		Severe*		Inhibitor	
N	*127*	*1*	*58*	*15*	*86*	*79*	*6*	*3*
Mean	*0.8*	*3.0*	*4.7*	*4.3*	***20.8***	***6.2***	*11.3*	*14.0*
Median (min – max)	*0 (0 – 12)*	*3*	*1 (0 – 31)*	*3 (1 – 16)*	***15 (0 – 144)***	***5 (0 – 31)***	*5.5 (1 – 32)*	*11 (5 – 26)*
persons on permanent prophylaxis	***1 (0.7%)***		***17 (20.7%)***		***89 (42.2%)***		***3 (33.3%)***	
% of drugs (FVIII and FIX) consumed by persons on permanent prophylaxix	***0.9 %***		***7.5 %***		***53.4 %***		***2.1 %***	

** without inhibitor*

Bleeding requiring treatment according to prophylaxis

Children
N=201



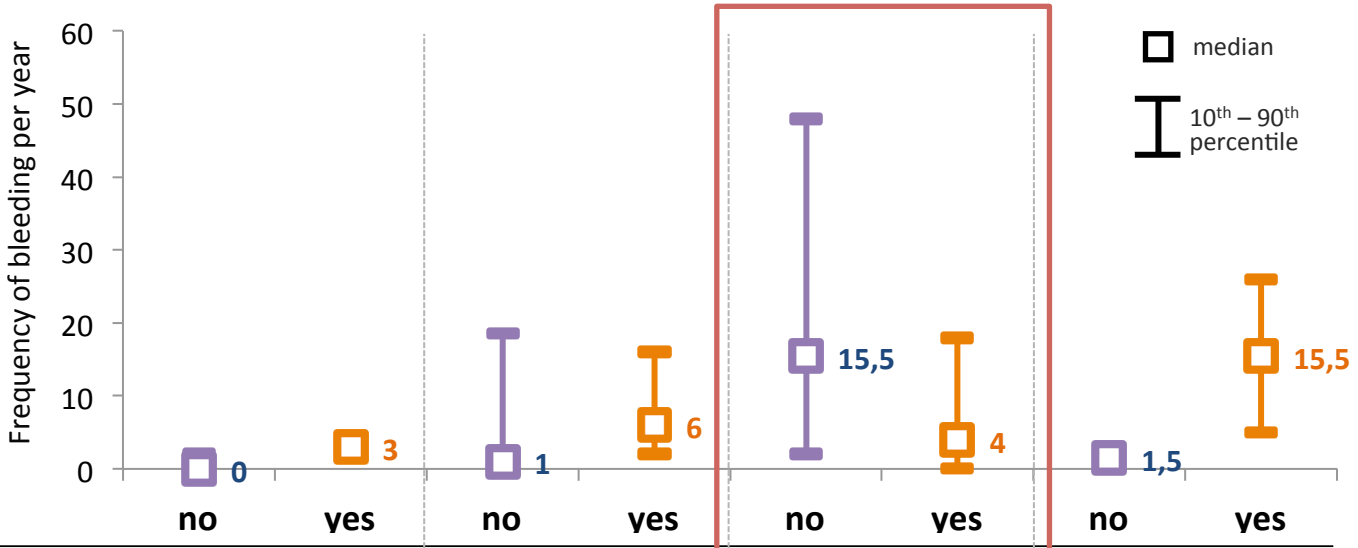
permanent prophylaxis

Frequency of bleeding	Mild*	Moderate*		Severe*		Inhibitor	
N	*77*	*38*	*9*	*18*	*49*	*4*	*1*
Mean	*0.8*	*3.9*	*2.2*	***13.2***	***6.1***	*16.3*	*11.0*
Median (min – max)	*0 (0 – 12)*	*1 (0 – 30)*	*2 (1 – 4)*	***7.5 (0 – 49)***	***5 (0 – 25)***	*15 (3 – 32)*	*11*
children on permanent prophylaxis	***0 (0%)***	***9 (19.1%)***		***51 (72.9%)***		***1 (20%)***	
% of drugs (FVIII and FIX) consumed by children on permanent prophylaxix	***0 %***	***9 %***		***70 %***		***4 %***	

** without inhibitor*

Bleeding requiring treatment according to prophylaxis

Adults
N=252

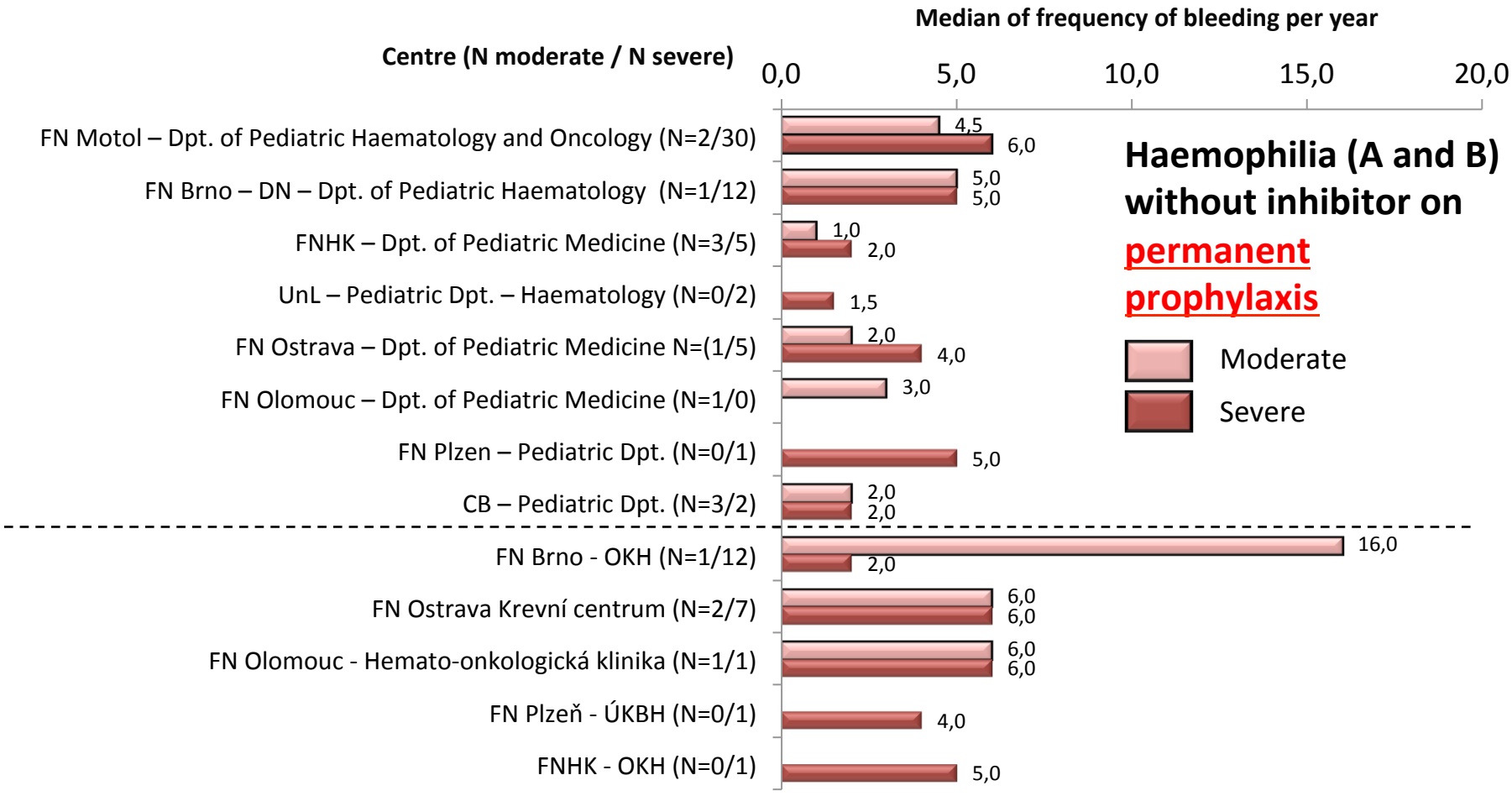


Frequency of bleeding	Mild*		Moderate*		Severe*		Inhibitor	
N	50	1	20	6	68	30	2	2
Mean	0.8	3.0	6.3	7.5	22.8	6.3	1.5	15.5
Median (min – max)	0 (0 – 7)	3	1 (0 – 31)	6 (2 – 16)	15.5 (0–144)	4 (0 – 31)	1.5 (1 – 2)	15.5 (5 – 26)
adults on permanent prophylaxis	1 (1.7%)		8 (22.9%)		38 (27%)		2 (50%)	
% of drugs (FVIII and FIX) consumed by adults on permanent prophylaxix	1.4 %		6.5 %		42.8 %		0.9 %	

* without inhibitor

Bleeding requiring treatment according to centres

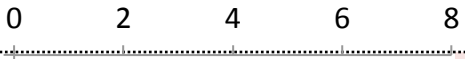
All
N=453



Bleeding requiring treatment according to centres

Children
N=201

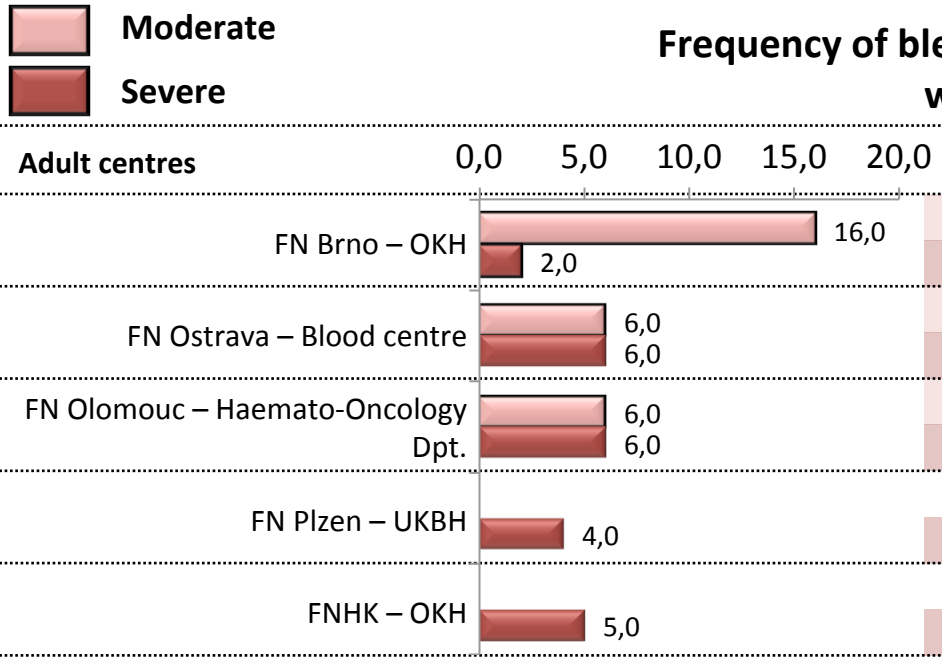
Frequency of bleeding in children with haemophilia (A and B) without inhibitor on permanent prophylaxis



Child centres	N	Mean	Median	Min	Max	Severity
FN Motol – Dpt. of Pediatric Haematology and Oncology	2	4.5	**4.5**	3	6	Moderate
	30	7.6	**6.0**	0	31	Severe
FN Brno – DN – Dpt. of Pediatric Haematology	1	5.0	**5.0**			Moderate
	12	5.3	**5.0**	0	13	Severe
FNHK – Dpt. of Pediatric Medicine	3	1.3	**1.0**	1	2	Moderate
	5	5.6	**2.0**	0	21	Severe
UnL – Pediatric Dpt. – Haematology	2	1.5	**1.5**	1	2	Severe
FN Ostrava – Dpt. of Pediatric Medicine	1	2.0	**2.0**			Moderate
	5	6.6	**4.0**	1	20	Severe
FN Olomouc – Dpt. of Pediatric Medicine	1	3.0	**3.0**			Moderate
FN Plzen – Pediatric Dpt.	1	5.0	**5.0**			Severe
CB – Pediatric Dpt.	3	2.7	**2.0**	2	4	Moderate
	2	2.0	**2.0**	1	3	Severe

Bleeding requiring treatment according to centres

Adults
N=252



Frequency of bleeding in adults with haemophilia (A and B) without inhibitor on permanent prophylaxis

Adult centres	N	Mean	Median	Min	Max	Severity
FN Brno – OKH	1	16.0	**16.0**			Moderate
	12	5.0	**2.0**	0	20	Severe
FN Ostrava – Blood centre	2	6.0	**6.0**	2	10	Moderate
	7	7.1	**6.0**	2	14	Severe
FN Olomouc – Haemato-Oncology Dpt.	1	6.0	**6.0**			Moderate
	1	6.0	**6.0**			Severe
FN Plzen – UKBH	1	4.0	**4.0**			Severe
FNHK – OKH	1	5.0	**5.0**			Severe

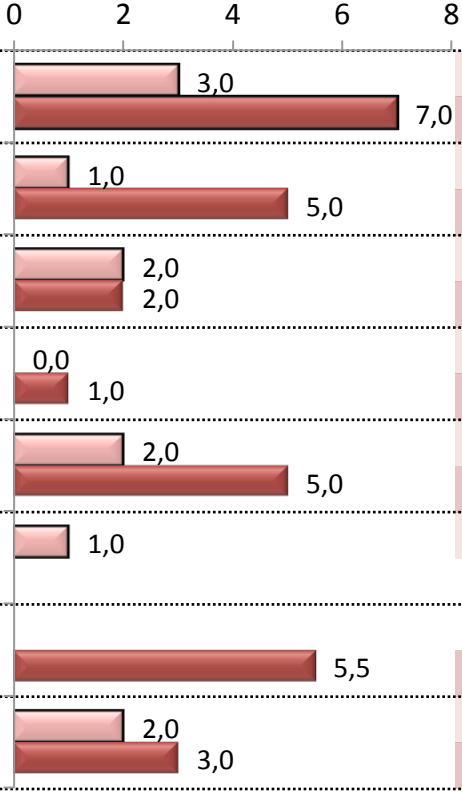
Bleeding requiring treatment according to centres

Children
N=201

Moderate

Severe

Frequency of bleeding in children with haemophilia (A and B) without inhibitor regardless of prophylaxis



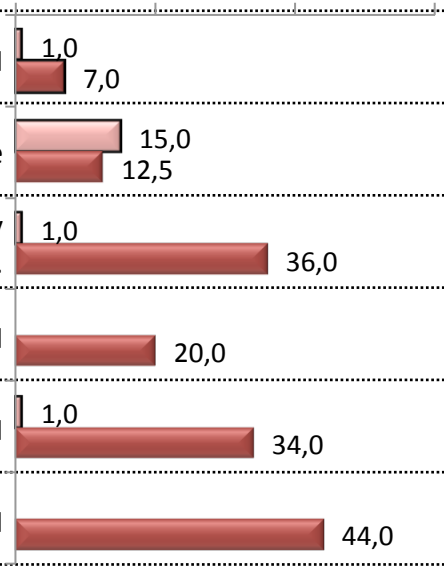
Child centres	N	Mean	Median	Min	Max	% on premanent prophylaxis
FN Motol – Dpt. of Pediatric Haematology and Oncology	16	6.1	**3.0**	0	30	**12.5**
	41	8.9	**7.0**	0	31	**73.2**
FN Brno – DN – Dpt. of Pediatric Haematology	8	1.3	**1.0**	0	5	**12.5**
	15	6.0	**5.0**	0	14	**80.0**
FNHK – Dpt. of Pediatric Medicine	9	1.8	**2.0**	0	3	**33.3**
	5	5.6	**2.0**	0	21	**100.0**
UnL – Pediatric Dpt. – Haematology	2	0.0	**0.0**	0	0	**0.0**
	3	1.3	**1.0**	1	2	**66.7**
FN Ostrava – Dpt. of Pediatric Medicine	9	4.8	**2.0**	0	19	**11.1**
	8	11.5	**5.0**	0	36	**62.5**
FN Olomouc – Dpt. of Pediatric Medicine	3	1.3	**1.0**	0	3	**33.3**
FN Plzen – Pediatric Dpt.	4	15.3	**5.5**	1	49	**25.0**
CB – Pediatric Dpt.	3	2.7	**2.0**	2	4	**100.0**
	3	3.3	**3.0**	1	6	**66.7**

Bleeding requiring treatment according to centres

Adults
N=252

Moderate
Severe

Frequency of bleeding in adults with haemophilia (A and B) without inhibitor regardless of prophylaxis



Adult centres	N	Mean	Median	Min	Max	% on premanent prophylaxis
FN Brno – OKH	11	4.2	**1.0**	0	16	**9.1**
	35	9.0	**7.0**	0	28	**34.3**
FN Ostrava – Blood centre	7	14.4	**15.0**	2	31	**28.6**
	24	16.5	**12.5**	2	64	**29.2**
FN Olomouc – Haemato-Oncology Dpt.	5	2.2	**1.0**	1	6	**20.0**
	9	29.2	**36.0**	1	64	**11.1**
FN Plzen – UKBH	8	17.0	**20.0**	4	25	**12.5**
FNHK – OKH	1	1.0	**1.0**			**0.0**
	6	59.8	**34.0**	5	144	**16.7**
UnL – OKH	4	39.5	**44.0**	20	50	**0.0**

All
N=453

Consumption of drugs

	Drug	Total annual consumption	Number of treated persons	Average annual consumption per treated person	Number of examined persons	Average annual consumption per examined person
FVIII	Immunate	6 503 750 IU	100	65 037.5 IU	386	16 849.1 IU
	Fanhdi	5 757 000 IU	66	87 227.3 IU		14 914.5 IU
	Octanate	1 558 000 IU	17	91 647.1 IU		4 036.3 IU
	Haemoctin	354 500 IU	3	118 166.7 IU		918.4 IU
	Advate	2 863 369 IU	38	75 351.8 IU		7 418.1 IU
	Kogenate	2 724 325 IU	33	82 555.3 IU		7 057.8 IU
	Recombinate	1 989 500 IU	18	110 527.8 IU		5 154.1 IU
	Other recombinant	290 500 IU	4	72 625.0 IU		752.6 IU
	FVIII celkem	**22 040 944 IU**	**265**	**83173.4 IU**		**57 100.9 IU**
FIX	Immunine	1 501 200 IU	32	46 912.5 IU	67	22 406.0 IU
	Octanine	746 600 IU	12	62 216.7 IU		11 143.3 IU
	Other plasma-derived	77 400 IU	1	77 400.0 IU		1 155.2 IU
	Other recombinant	379 916 IU	2	189 958.0 IU		5 670.4 IU
	FIX celkem	**2 705 116 IU**	**43**	**62 909.7 IU**		**40 374.9 IU**
aPCC	Feiba	60 000 IU	1	60 000.0 IU		
rFVIIa	NovoSeven	805 mg	5	161.0 mg		
Plasma-derived factors - TOTAL*		**17 508 450 IU**	**237**	**73 875.3 IU**	453	**38 517.5 IU**
Recombinant factors - TOTAL*		**8 247 610 IU**	**92**	**89 647.9 IU**		**18 206.6 IU**
TOTAL CONSUMTION *		**25 756 060 IU**	**320**	**80 487.7 IU**		**56 856.6 IU**

- 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

Consumption of drugs

Children
N=201

	Drug	Total annual consumption	Number of treated persons	Average annual consumption per treated child	Number of examined children	Average annual consumption per examined child
FVIII	Immunate	2 000 250 IU	37	54 060.8 IU	175	11 430.0 IU
	Fanhdi	1 099 750 IU	13	84 596.2 IU		6 284.3 IU
	Octanate	1 222 500 IU	11	111 136.4 IU		6 985.7 IU
	Haemoctin	163 500 IU	1	163 500.0 IU		934.3 IU
	Advate	1 775 712 IU	30	59 190.4 IU		10 146.9 IU
	Kogenate	1 853 825 IU	26	71 301.0 IU		10 593.3 IU
	Recombinate	464 000 IU	3	154 666.7 IU		2 651.4 IU
	Other recombinant	290 500 IU	4	72 625.0 IU		1 660.0 IU
	FVIII celkem	**8 870 037 IU**	**117**	**75 812.3 IU**		**50 685.9 IU**
FIX	Immunine	517 200 IU	9	57 466.7 IU	26	19 892.3 IU
	Octanine	273 500 IU	7	39 071.4 IU		10 519.2 IU
	Other plasma-derived					
	Other recombinant					
	FIX celkem	**790 700 IU**	**15**	**52 713.3 IU**		**30 411.5 IU**
aPCC	Feiba	60 000 IU	1	60 000.0 IU		
rFVIIa	NovoSeven	629 mg	4	157.3 mg		
Plasma-derived factors - TOTAL*		**5 336 700 IU**	**77**	**69 307.8 IU**	201	**26 252.2 IU**
Recombinant factors - TOTAL*		**4 384 037 IU**	**60**	**73 067.3 IU**		**21 811.1 IU**
TOTAL CONSUMTION *		**9 720 737 IU**	**133**	**73 088.2 IU**		**48 361.9 IU**

- 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
N=252

Consumption of drugs

	Drug	Total annual consumption	Number of treated persons	Average annual consumption per treated person	Number of examined adults	Average annual consumption per examined adult
	Immunate	4 503 500 IU	63	71 484.1 IU		21 343.6 IU
	Fanhdi	4 657 250 IU	53	87 872.6 IU		22 072.3 IU
	Octanate	335 500 IU	6	55 916.7 IU		1 590.0 IU
	Haemoctin	191 000 IU	2	95 500.0 IU		905.2 IU
FVIII	Advate	1 087 657 IU	8	135 957.1 IU	211	5 154.8 IU
	Kogenate	870 500 IU	7	124 357.1 IU		4 125.6 IU
	Recombinate	1 525 500 IU	15	101 700.0 IU		7 229.9 IU
	Other recombinant					
	FVIII celkem	**13 170 907 IU**	**148**	**88 992.6 IU**		**62 421.4 IU**
	Immunine	984 000 IU	23	42 782.6 IU		24 000.0 IU
	Octanine	473 100 IU	5	94 620.0 IU		11 539.0 IU
FIX	Other plasma-derived	77 400 IU	1	77 400.0 IU	41	1 887.8 IU
	Other recombinant	379 916 IU	2	189 958.0 IU		9 266.2 IU
	FIX celkem	**1 914 416 IU**	**28**	**68 372.0 IU**		**46 693.1 IU**
aPCC	Feiba					
rFVIIa	NovoSeven	176 mg	1	176 mg		
Plasma-derived factors - TOTAL*		**12 171 750 IU**	**160**	**76 073.4 IU**	252	**48 300.6 IU**
Recombinant factors - TOTAL*		**3 863 573 IU**	**32**	**120 736.7 IU**		**15 331.6 IU**
TOTAL CONSUMTION *		**16 035 323 IU**	**187**	**85 750.4 IU**		**63 632.2 IU**

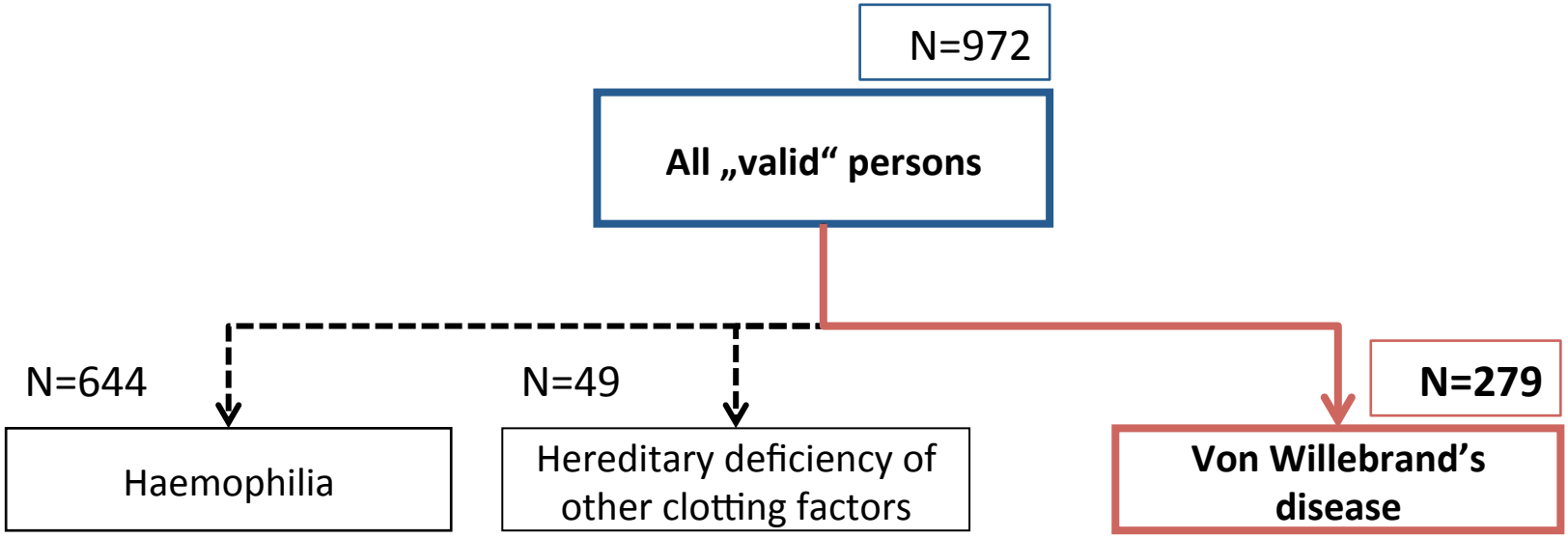
- plasma-derived factors = Immunate, Fanhdi, Octanate, Immunine, Octanine, Other plasma-derived
- recombinant factors = Advate, Kogenate, Recombinate, BAX 326, Other recombinant

*TOTAL CONSUMPTION = all mentioned drugs excluding Feiba and NovoSeven

Part B

Persons with Von Willebrand’s disease

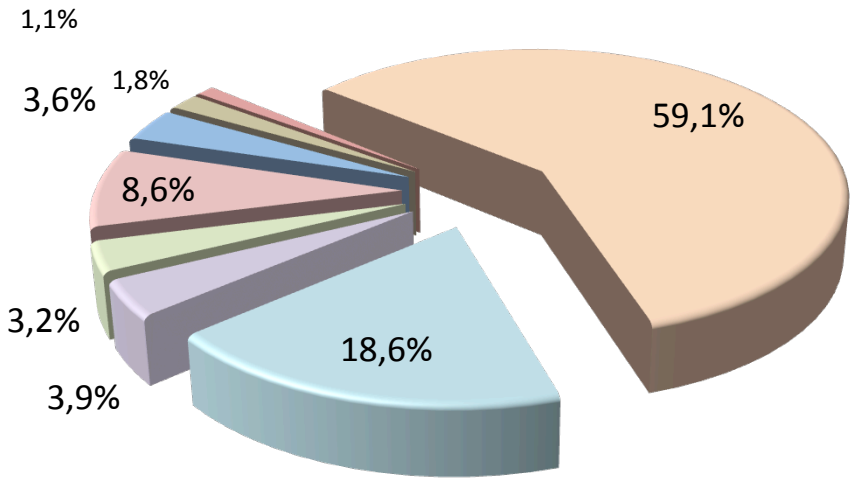
Sample size



Cca 1000 symptomatic vWDs should be in CZ
We know about 400 patients
279 of them are in CNHP registry so far

Number of patients in participating centres

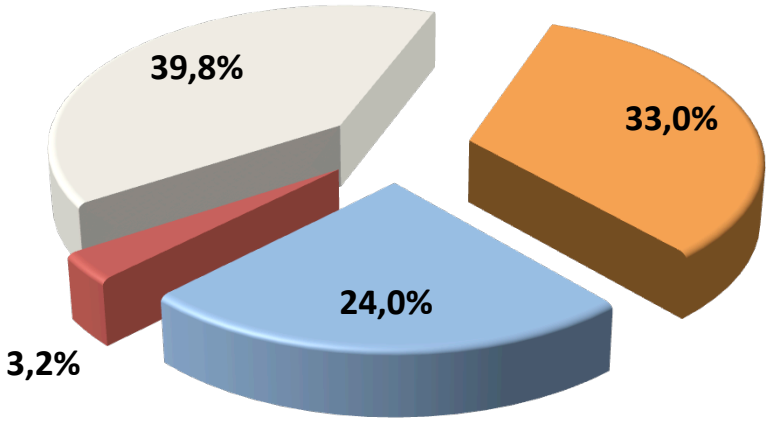
N=279



Centrum	Patients N	Patients %
FN Brno – OKH	165	59.1
FN Ostrava – Blood centre	52	18.6
FN Olomouc – Haemato-Oncology Dpt.	11	3.9
KN Liberec – OKH	9	3.2
FN Plzen – Pediatric Dpt.	24	8.6
FN Motol – Dpt. of Pediatric Haematology and Oncology	10	3.6
UnL – Pediatric Dpt. – Haematology	5	1.8
FN Brno – DN – Dpt. of Pediatric Haematology	3	1.1
Total	279	100.0

Type of Von Willebrand's disease

N=279

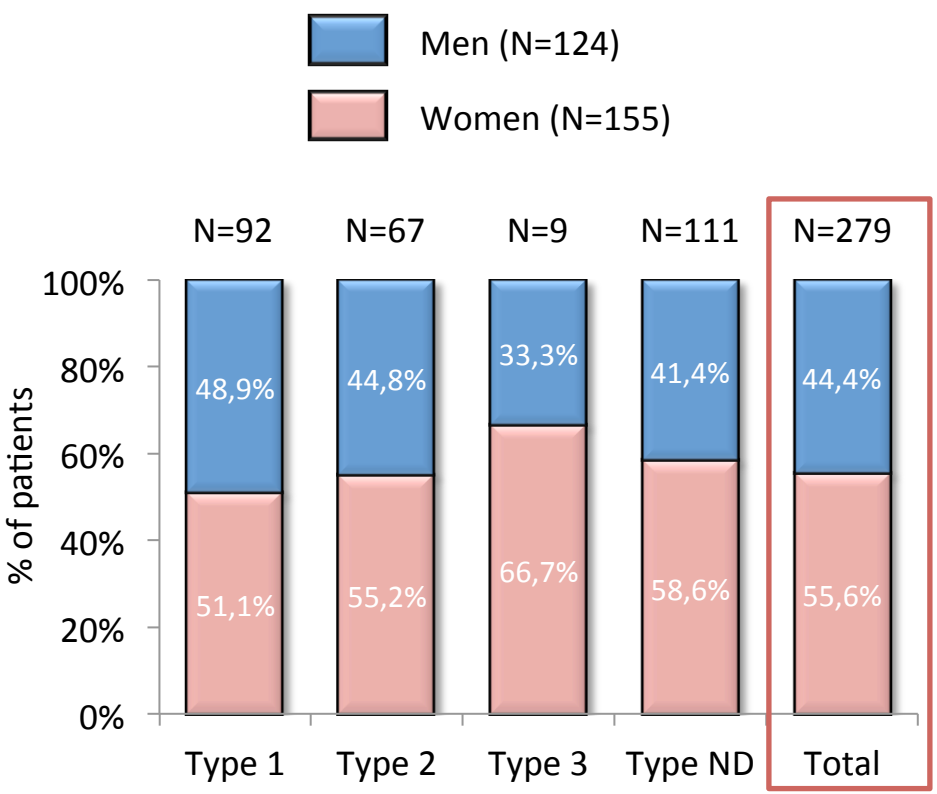


Type of disease	Patients N	Patients %
Type 1	92	33.0
Type 2	67	24.0
Type 2A	*41*	*14.7*
Type 2B	*5*	*1.8*
Type 2M	*18*	*6.5*
Type 2N	*3*	*1.1*
Type 3	9	3.2
Type not determined	111	39.8
Total	279	100.0

Sex and age of patients

N=279

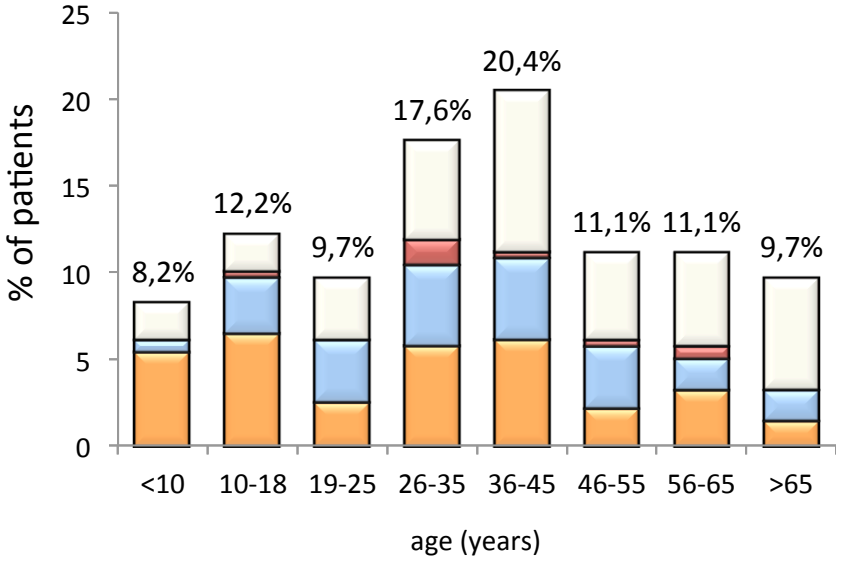
Sex



Type ND = not determined

Current age*

	Type 1	*Type 2*	*Type 3*	*Type ND*	**Total**
N	*92*	*67*	*9*	*111*	**279**
Mean	*30.4*	*36.2*	*38.3*	*43.0*	**37.0**
Median	*29*	*35*	*34*	*42*	**36**
min - max	*1 – 84*	*5-75*	*16-61*	*1-86*	**1-86**

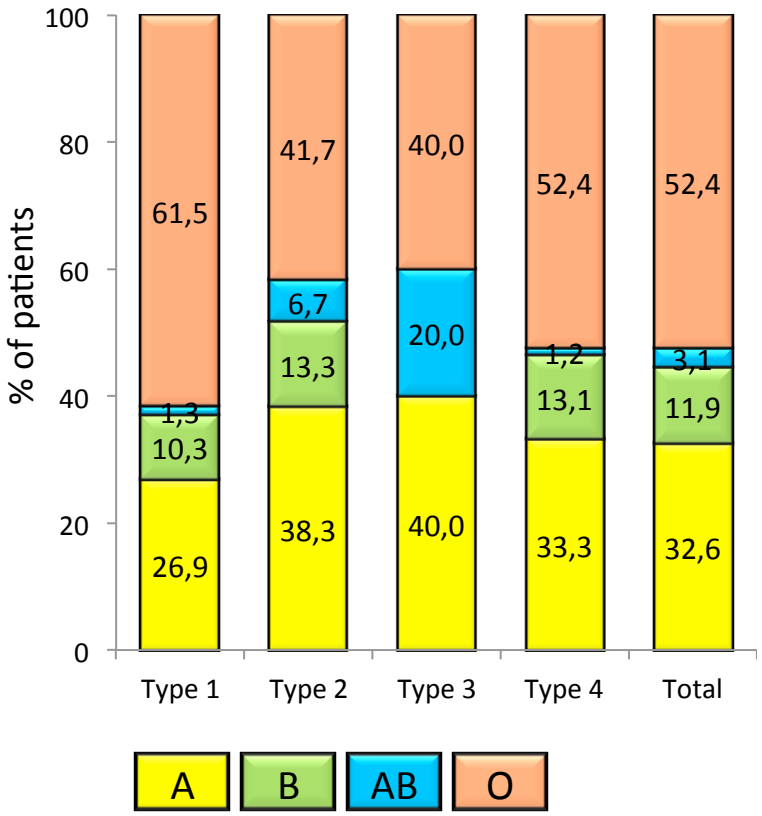


** age reached in year 2012*

Czech National Hemophilia Program

Blood group

N=279

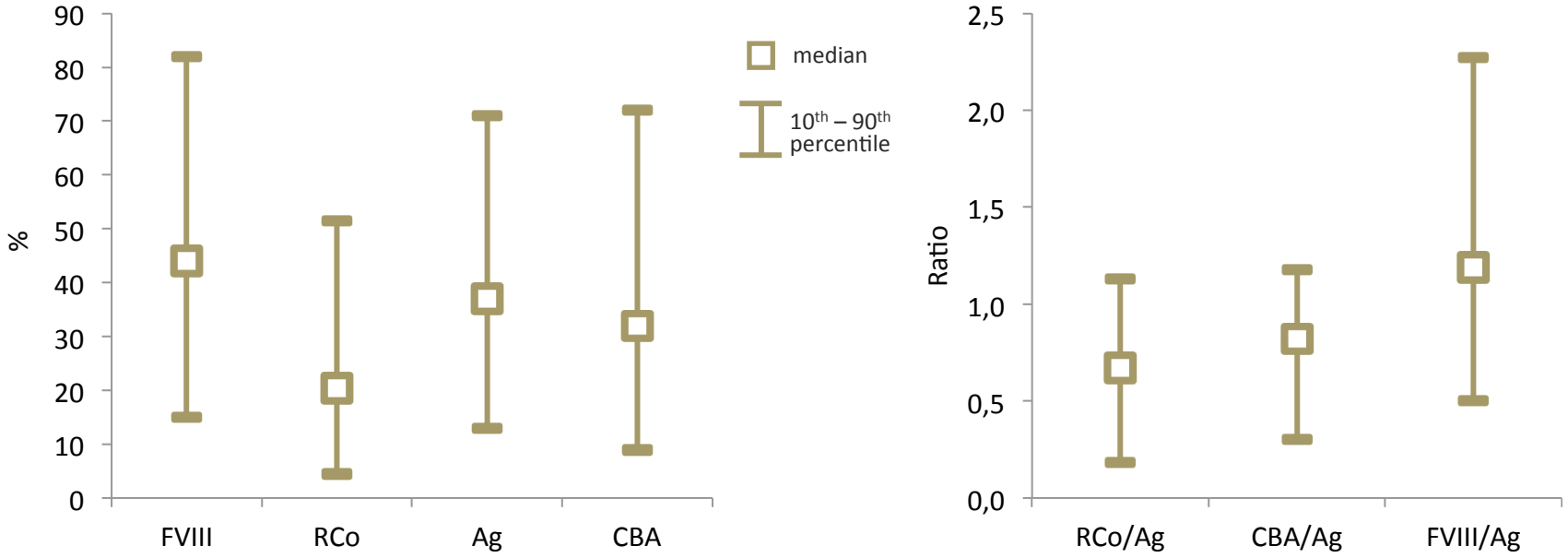


Blood group		A	B	AB	O	ND	Total N	Valid N
	N	*21*	*8*	*1*	*48*	*14*	*92*	*78*
Type 1	*%[1]*	*22.8*	*8.7*	*1.1*	*52.2*	*15.2*	*100*	
	%[2]	*26.9*	*10.3*	*1.3*	*61.5*			*100*
	N	*23*	*8*	*4*	*25*	*7*	*67*	*60*
Type 2	*%[1]*	*34.3*	*11.9*	*6*	*37.3*	*10.4*	*100*	
	%[2]	*38.3*	*13.3*	*6.7*	*41.7*			*100*
	N	*2*	*0*	*1*	*2*	*4*	*9*	*5*
Type 3	*%[1]*	*22.2*	*0*	*11.1*	*22.2*	*44.4*	*100*	
	%[2]	*40*	*0*	*20*	*40*			*100*
	N	*28*	*11*	*1*	*44*	*27*	*111*	*84*
Type ND	*%[1]*	*25.2*	*9.9*	*0.9*	*39.6*	*24.3*	*100*	
	%[2]	*33.3*	*13.1*	*1.2*	*52.4*			*100*
	N	74	27	7	119	52	279	227
Total	*%[1]*	26.5	9.7	2.5	42.7	18.6	100	
	%[2]	**32.6**	**11.9**	**3.1**	**52.4**			**100**

%[1] - % of total N
%[2] - % of valid N

Factor levels

N=279

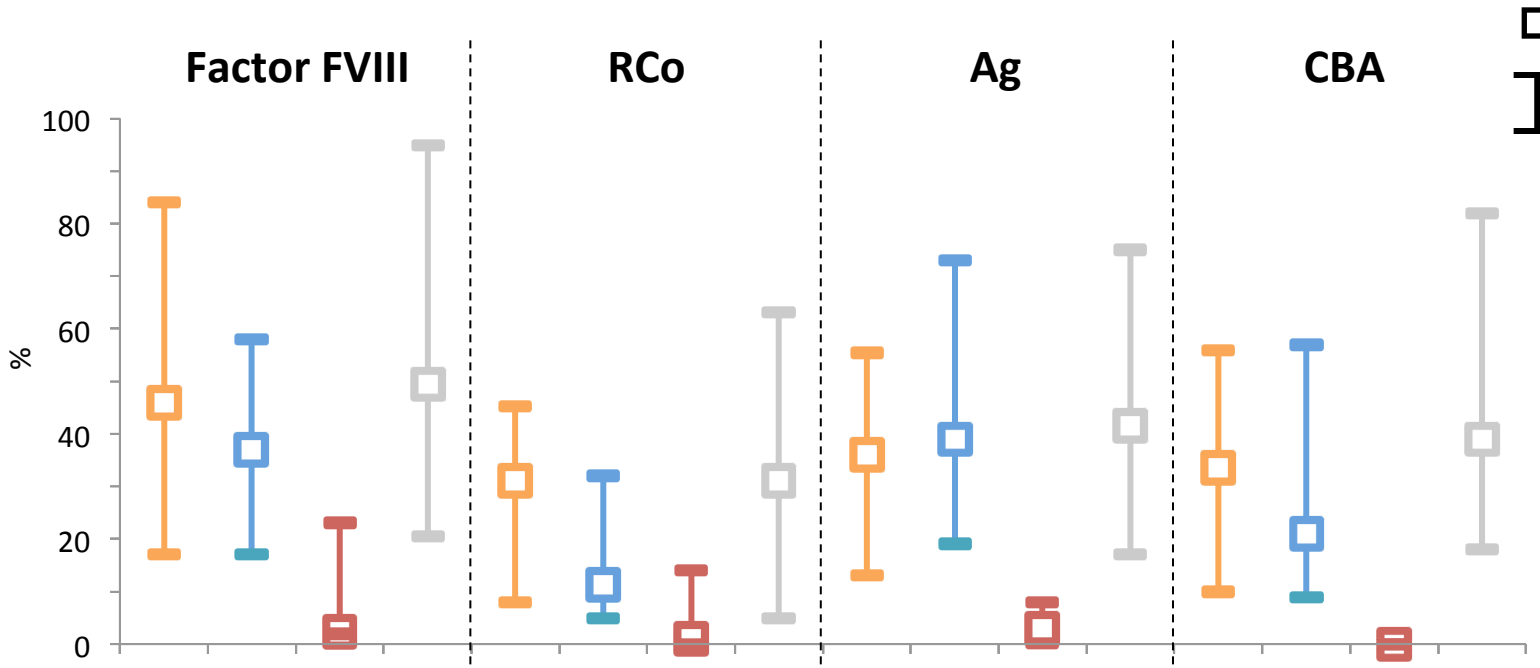


Factor FVIII*	RCo	Ag	CBA		RCo/Ag	CBA/Ag	FVIII/Ag
265	*260*	*259*	*118*	**N**	*255*	*117*	*257*
47.5	*26.0*	*39.9*	*36.1*	**Mean**	*0.72*	*0.87*	*1.74*
44 (0–219)	*20.5 (0–135)*	*37 (0–177)*	*32 (0–127)*	**Median (min – max)**	*0.67 (0–13.9)*	*0.82 (0–11.1)*	*1.19 (0–42.4)*

** Factor FVIII was assessed by coagulati on method in 249 patients, by chromogenic method in 15 patients and method is missing in 1 patient*

Factor levels according to type of VWD disease I.

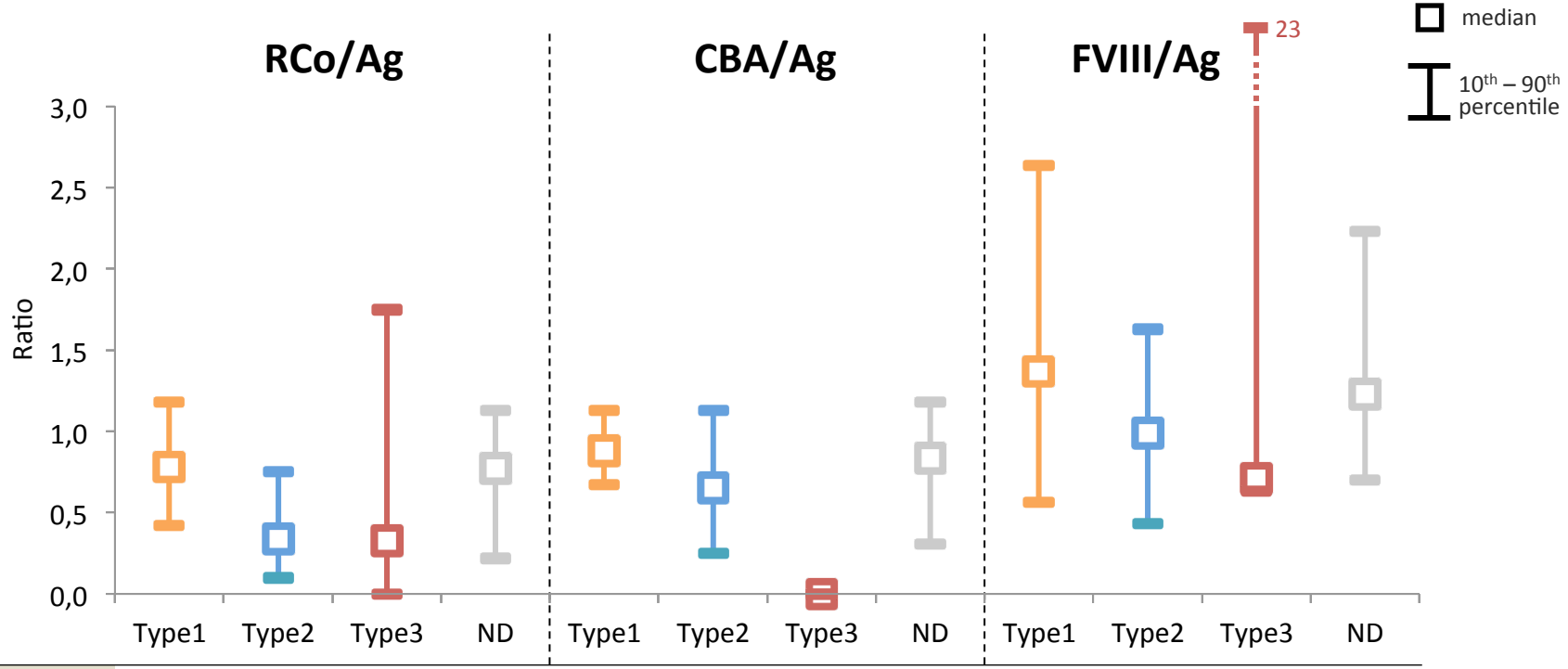
N=279



	Factor FVIII				RCo				Ag				CBA			
	Type1	Type2	Type3	ND	Type1	Type2	Type3	ND	Type1	Type2	Type3	ND	Type1	Type2	Type3	ND
N	91	65	9	100	90	65	9	96	90	64	9	96	32	38	4	44
Mean	48.4	38.6	4.9	56.4	28.0	15.3	2.4	33.6	35.3	43.6	3.1	45.2	35.0	29.4	0.0	46.0
Median	46.0	37.0	2.3	49.5	31.1	11.3	1.0	31.0	36.0	39.0	3.0	41.5	33.5	21.0	0.0	39.0

Factor levels according to type of VWD disease II.

N=279



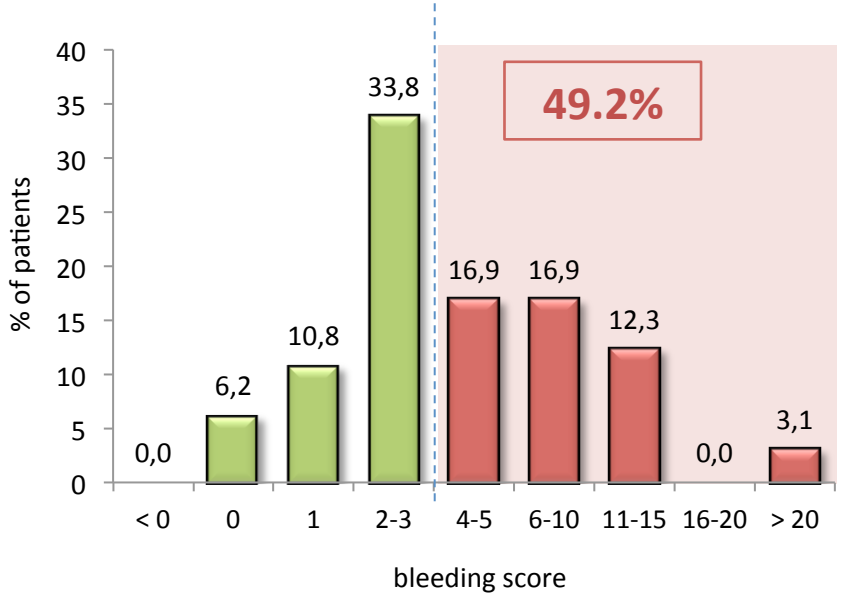
	RCo/Ag				CBA/Ag				FVIII/Ag			
	Type1	Type2	Type3	ND	Type1	Type2	Type3	ND	Type1	Type2	Type3	ND
N	89	64	9	93	32	37	4	44	90	64	9	94
Mean	0.92	0.40	0.58	0.77	1.21	0.67	0.00	0.85	1.97	1.02	3.69	1.81
Median	0.78	0.34	0.33	0.77	0.88	0.65	0.00	0.84	1.37	0.99	0.71	1.23

Bleeding score according to sex

N=143*

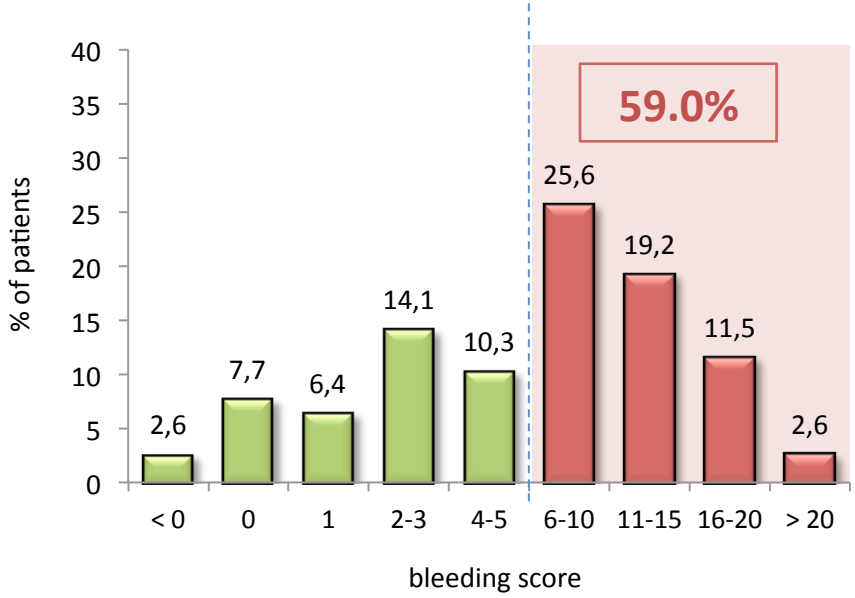
Men

N	*65*
Mean	*5.0*
Median (min - max)	*3 (0 – 25)*



Women

N	*78*
Mean	*7.7*
Median (min - max)	*6 (-3 – 23)*



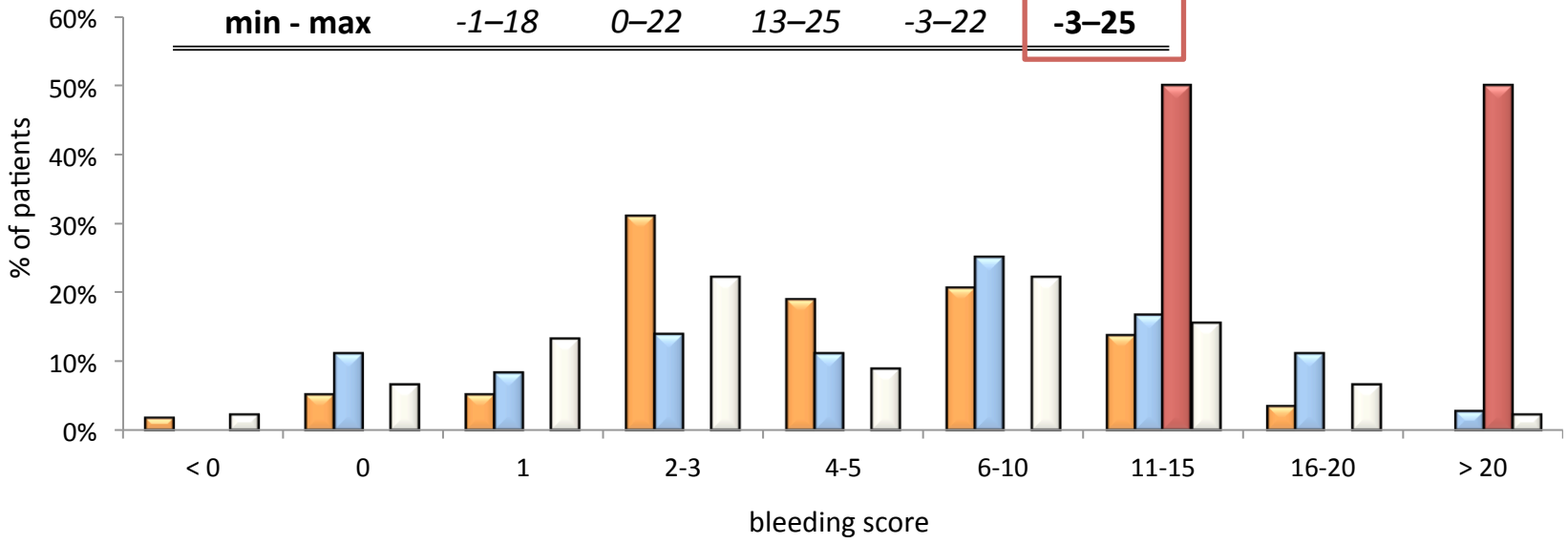
** Missing information on bleeding score in 136 patients.*

Bleeding score according to type of disease

N=143*

Bleeding score

	Type 1	Type 2	Type 3	Type ND	Total
N total	*92*	*67*	*9*	*111*	**279**
N valid	*58*	*36*	*4*	*45*	**143**
Mean	***5.3***	***7.4***	***18.8***	***6.2***	**6.5**
Median	***4.5***	***6.0***	***18.5***	***4.0***	**5.0**
min - max	*-1–18*	*0–22*	*13–25*	*-3–22*	**-3–25**

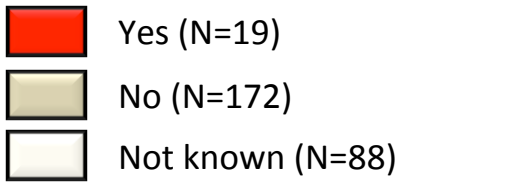


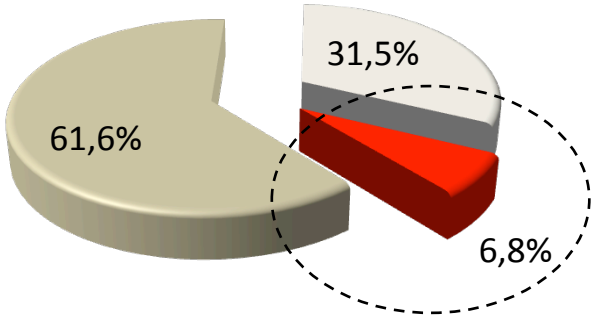
** Missing information on bleeding score in 136 patients.*

Other diseases

N=279

Experienced hepatitis





9x hepatitis A
5x hepatitis B
2x hepatitis A+B
3x hepatitis C

None of the patients is HIV positive.

Consumption of drugs in year 2012

N=279

	Number of treated patients total (type1/type2/type3/typeND)	Total annual consumption	Average annual consumption per treated patient
Fanhdi	**6 (3/2/0/1)**	**11 000 IU**	**1 833.3 IU**
Haemate P	**26 (7/9/4/6)**	**406 000 IU**	**15 615.4 IU**
of them on prophylaxis	*1 (0/0/1/0)*	*12 500 IU*	*12 500.0 IU*
Immunate	**1 (1/0/0/0)**	**1 500 IU**	**1 500.0 IU**
Willfact	**1 (0/0/1/0)**	**148 000 IU**	**148 000.0 IU**
of them on prophylaxis	*1 (0/0/1/0)*	*148 000 IU*	*148 000.0 IU*
Total	**33 (11/10/5/7)**	**566 500 IU**	**17166.7 IU**
of them on prophylaxis	*2 (0/0/2/0)*	*160 000 IU*	*80 000.0 IU*
Total - type 1	11	72 000 IU	**6 545.5 IU**
Total - type 2	10	227 500 IU	**22 750.0 IU**
Total - type 3	5	172 500 IU	**34 500.0 IU**
Total - type ND	7	94 500 IU	**13 500.0 IU**