

The status of care for persons with haemophilia registered within CNHP registry Annual Report 2020

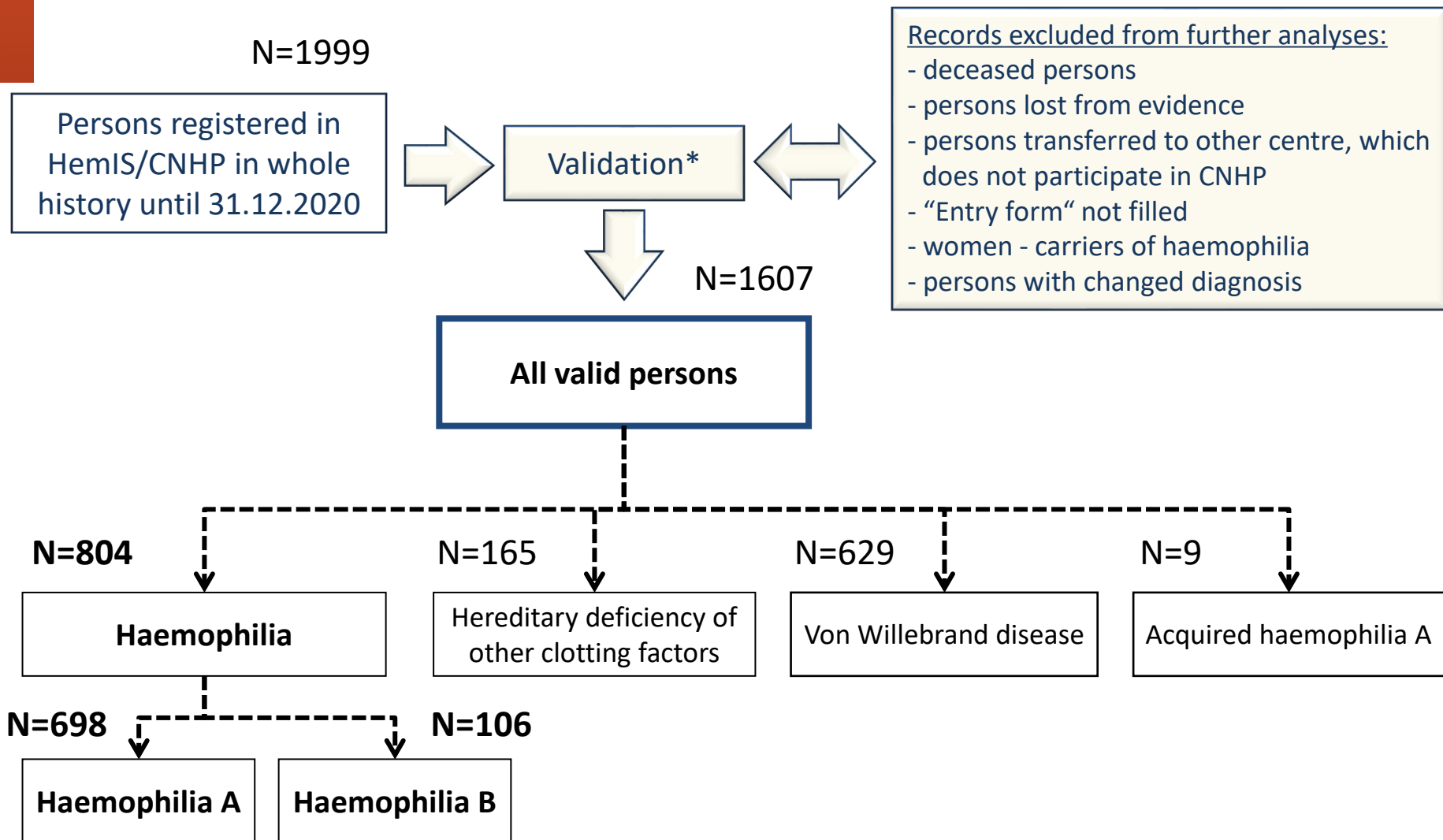
Jan Blatný, Petra Ovesná

on behalf of

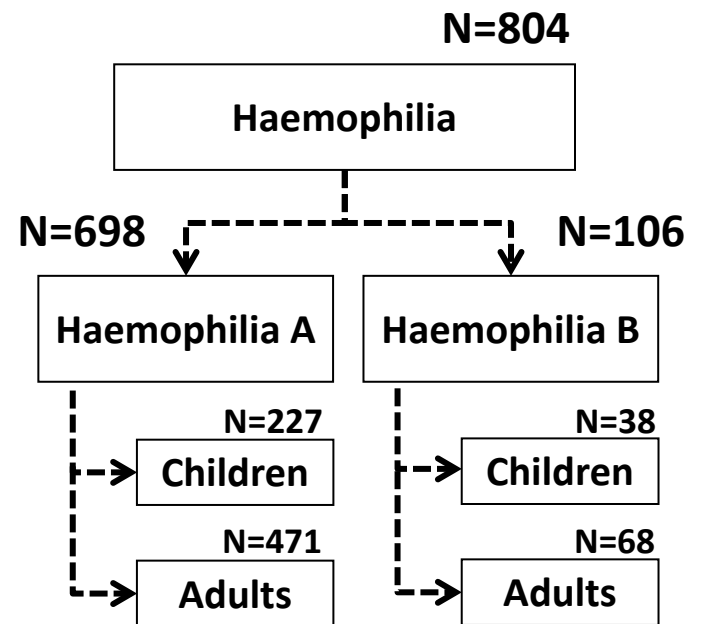
Centres contributing to CNHP registry
(Czech National Haemophilia Programme)

Export date: March 30, 2021

Sample size, valid records



Persons with haemophilia (PWH)



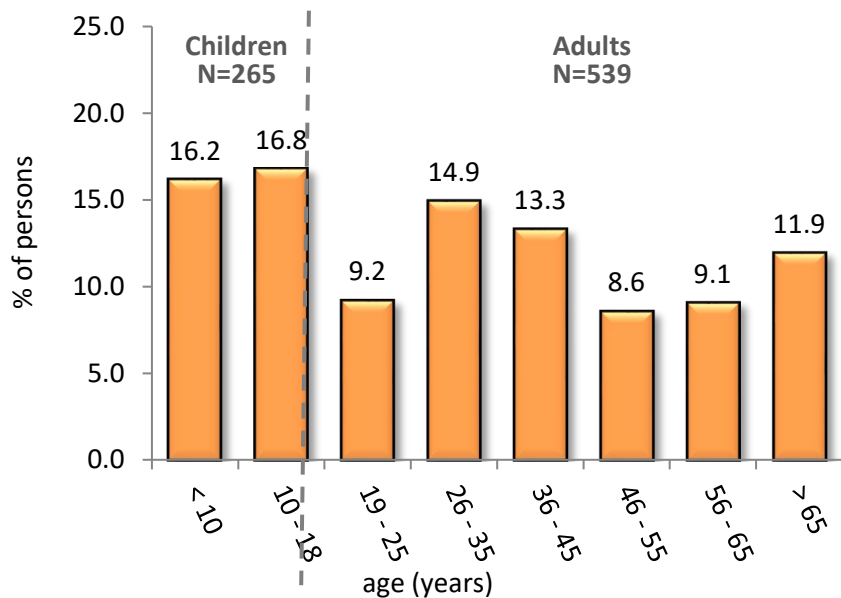
Centres participating in CNHP

Paediatric centres	Valid persons	
	N	%
Prague – Dpt. of Pediatric Haematology and Oncology, CUH Motol	95	11.8
Brno – Dpt. of Pediatric Haematology, CUH Brno	59	7.3
Hradec Králové – Dpt. of Pediatric Medicine, UH HK	33	4.1
Ostrava – Dpt. of Pediatric Medicine, UH Ostrava	24	3.0
Olomouc – Dpt. of Pediatric Medicine, UH Olomouc	20	2.5
Ústí n.L. – Pediatric Dpt. – Haematology, Masaryk Hospital	19	2.4
České Budejovice – Pediatric Dpt., Hospital CB	17	2.1
Pilsen – Pediatric Dpt., UH Pilsen	13	1.6

Adult centres	Valid persons	
	N	%
Brno – Dpt. Of Clin Hematol, UH Brno	165	20.5
Ostrava – Blood centre, UH Ostrava	76	9.5
Hradec Králové – IV. Internal and Hematology Dpt., UH HK	70	8.7
Olomouc – Haemato-Oncology Dpt., UH Olomouc	60	7.5
Pilsen – Dpt. of Biochemistry and Hematology, UH Pilsen	50	6.2
Liberec – Dpt. Of Clin Hematol, Hospital Liberec	41	5.1
Ústí n.L. – Dpt. Of Clin Hematol, Masaryk Hospital	28	3.5
České Budějovice – Dpt. Of Clin Hematol, Hospital CB	26	3.2
Pilsen - Hemacentrum	8	1.0

Basic demographics

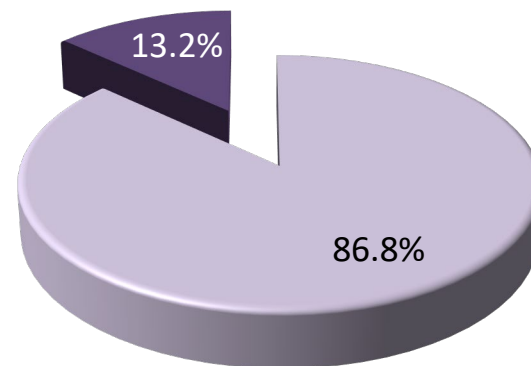
	Actual age* (years)
N	804
Mean	33.4
Median (min - max)	31 (0 – 95)



* age reached in year 2020

Type of haemophilia

- Haemophilia A (N=698)
- Haemophilia B (N=106)



Six children with haemophilia were born in 2020.

Persons with haemophilia and inhibitors in 2020

Active inhibitors were recorded in 19 persons in the end of year 2020

- 1 inhibitor in children with severe HA newly developed in 2020

PWH with inhibitors:

- 11 children and 8 adults
- 18 haemophilia A and 1 haemophilia B
- 16 in severe and 3 in moderate haemophilia
- 18 high-titre and 1 low-titre (<5BU)
- 12 high response and 5 low response inhibitors; this information not available in 2 PWH with inhibitors
- 15 patients were treated with emicizumab
 - 12 patients were treated only with emi and 3 patients with emi and rFVIIa during the year
- 1 patients was treated only with rFVIIa and 1 patient only with aPCC
 - 1 patient was without any „by-pass“ therapy or emi therapy and 1 patient was without any recorded treatment at all

ITT:

- Two patients have already been on-going ITT in 2020 (started earlier).
- No patient started ITI in 2020.
- No patient finished ITI during 2020 (successfully or unsuccessfully).

ABR and treatment regimens in patients with inhibitor

	Type	Year of birth	Severity	ITT	Emi prophylaxis	By-pass prophylaxis	Titre	Responder	ABR	Joint / other
1	HA	2018	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	HR	0	0/0
2	HA	2018	Severe	No/NA	Permanent	OD	Low	LR	0	0/0
3	HA	2017	Severe	Yes	Permanent	OD	High (>5 BU/ml)	HR	0	0/0
4	HA	2016	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	2	0/2
5	HA	2016	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0/0
6	HA	2015	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	HR	0	0/0
7	HA	2015	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0/0
8	HA	2011	Moderate	No/NA	Permanent	OD	High (>5 BU/ml)	HR	1	0/1
9	HA	2004	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	HR	0	0/0
10	HA	2003	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	HR	0	0/0
11	HA	2001	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	HR	0	0/0
12	HA	1977	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	HR	0	0/0
13	HA	1975	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	NA	1	1/0
14	HA	1971	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	HR	0	0/0
15	HA	1971	Severe	No/NA	OD	OD	High (>5 BU/ml)	LR	1	1/0
16	HA	1956	Severe	No/NA	OD	Permanent	High (>5 BU/ml)	HR	0	0/0
17	HA	1949	Moderate	No/NA	OD	OD	High (>5 BU/ml)	NA	NA	/
18	HA	1941	Moderate	Yes	Permanent	OD	High (>5 BU/ml)	HR	2	1/1
19	HB	2007	Severe	No/NA	OD	Permanent	High (>5 BU/ml)	HR	13	6/7

Severity

- Mild
- Moderate
- Severe

ITT

- Yes
- No/NA

By-pass/emi prophylaxis

- Permanent
- Temporary
- OD

Titre

- High (>5 BU/ml)
- Low

Responder

- HR
- LR

new in 2020

NA – not available

ABR according to treatment regimen in PWH with inhibitor

Diagnosis	ITT	Emi/by-pass prophylaxis	N	ABR (mean)	ABR (median, min-max)	Joint / other bleeds (median)
Haemophilia A	Yes	Emi permanent	2	1.00	1 (0-2)	0.5 / 0.5
	No	Emi permanent	13	0.31	0 (0-2)	0 / 0
		BPA permanent	1	0.00	0 (0-0)	0 / 0
		OD	1	1.00	1 (1-1)	1 / 0
Haemophilia B	No	BPA permanent	1	13.00	13 (13-13)	6 / 7

* ABR is missing in 1 adult.

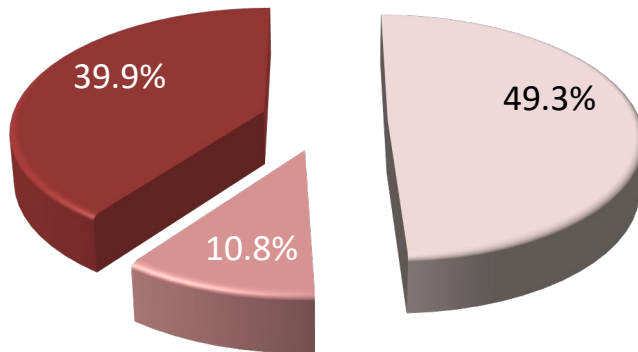
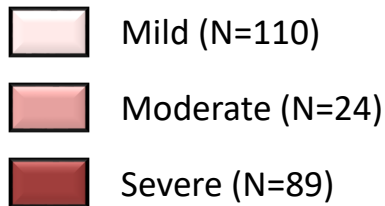
Demographic characteristics

Haemophilia A

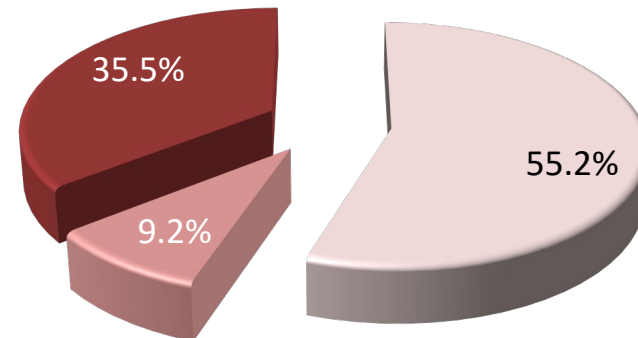
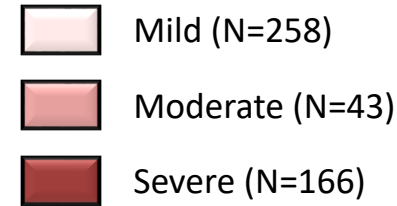


Severity of haemophilia A

Children (N=223*)



Adults (N=467*)

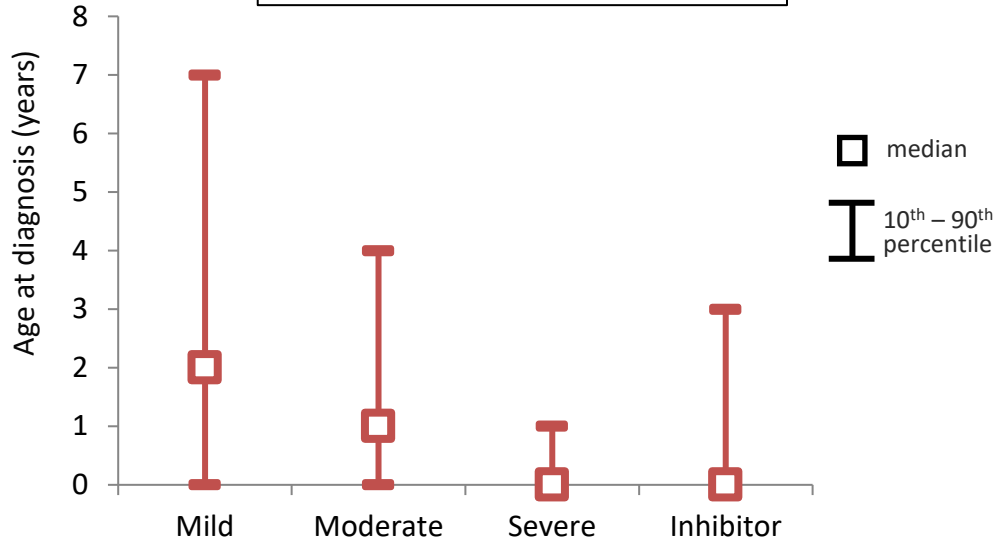


* Severity of haemophilia not known in 4 children and 4 adults with haemophilia A.

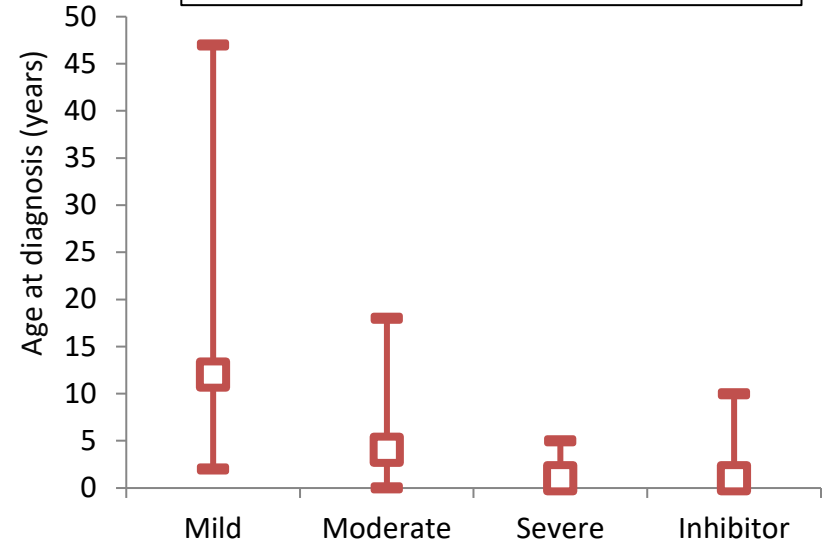
¹ severity of haemophilia not known in 4 children and 4 adults

Age at diagnosis according to severity of haemophilia A

Children (N=213²)



Adults (N=373³)



Mild*	Moderate*	Severe*	Inhibitor ⁺	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor ⁺
108	23	82	10	N valid	217	34	122	8
2.8	1.7	0.6	0.7	Mean	19.1	7.1	2.2	2.3
2 (0 – 17)	1 (0 – 10)	0 (0 – 7)	0 (0 – 4)	Median (min – max)	12 (0 – 68)	4 (0 – 32)	1 (0 – 38)	1 (0 – 10)

² Missing information on year of diagnosis in 10 children.

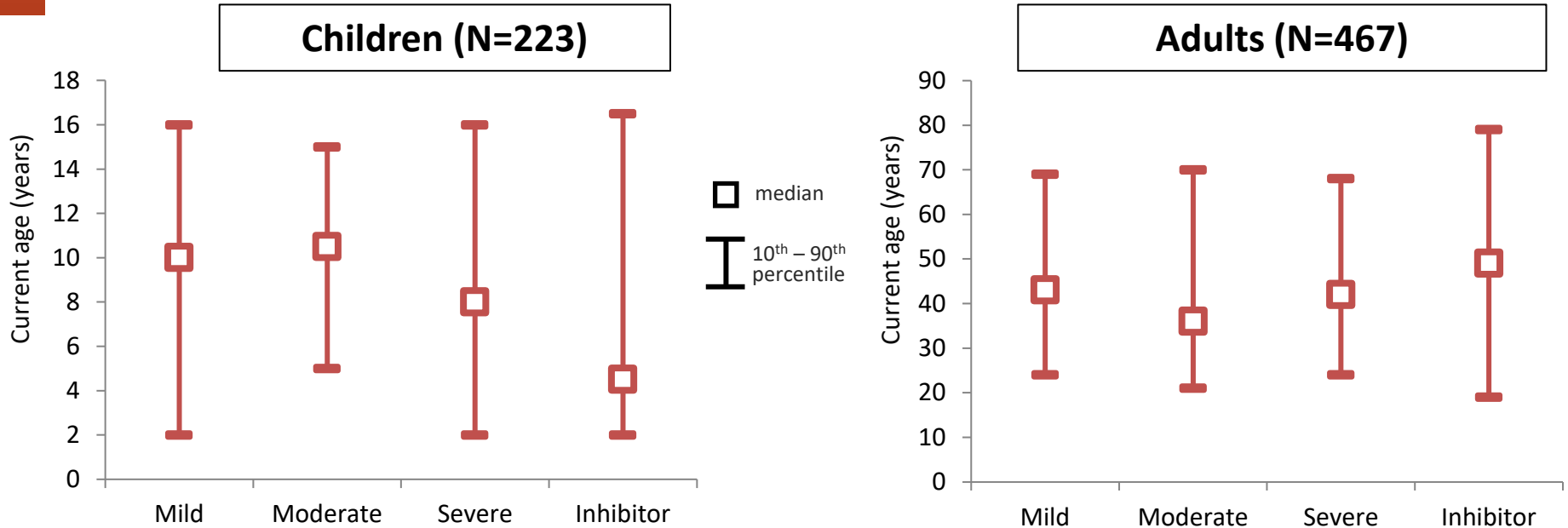
³ Missing information on year of diagnosis in 94 adults.

* including persons with inhibitor

⁺ in 2020

¹ severity of haemophilia not known in 4 children and 4 adults

Actual age according to severity of haemophilia A



Mild*	Moderate*	Severe*	Inhibitor ⁺	Current age ^{**} (years)	Mild*	Moderate*	Severe*	Inhibitor ⁺
110	24	89	10	N valid	258	43	166	8
9.6	10.3	8.7	6.7	Mean	45.6	42.6	44.2	52.4
10 (0 – 18)	10.5 (2 – 17)	8 (0 – 18)	4.5 (2 – 17)	Median (min – max)	43 (19 – 90)	36 (19 – 79)	42 (19 – 81)	49 (19 – 79)

* including persons with inhibitor

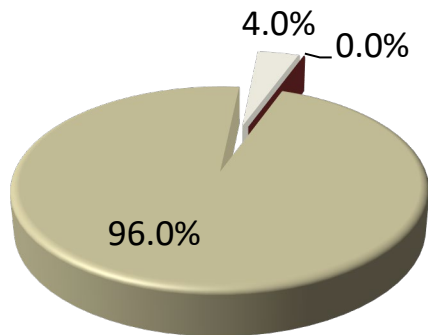
⁺ in 2020

^{**} age reached in year 2020

Hepatitis (ever) experienced

Experienced hepatitis

- Yes (N=0)
- No (N=218)
- Not known (N=9)



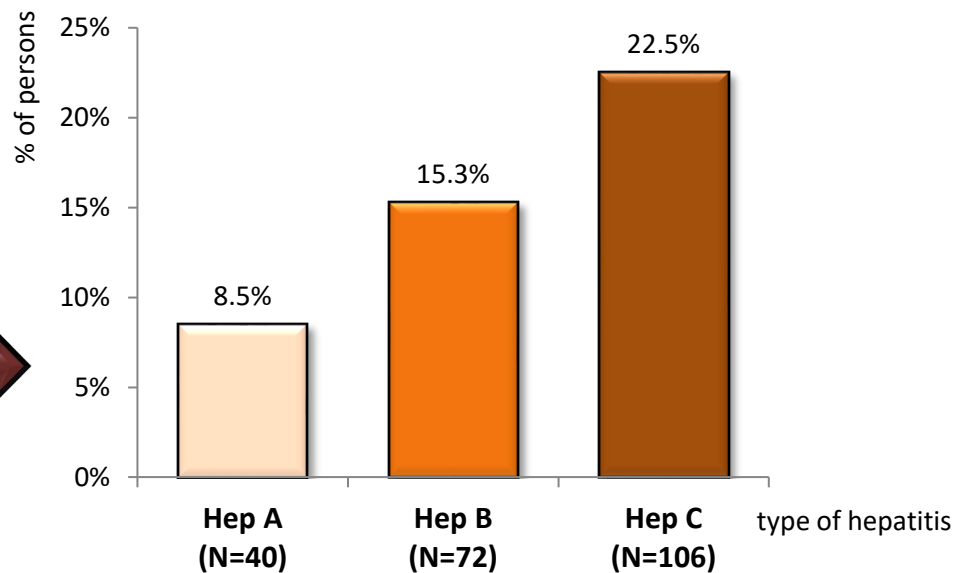
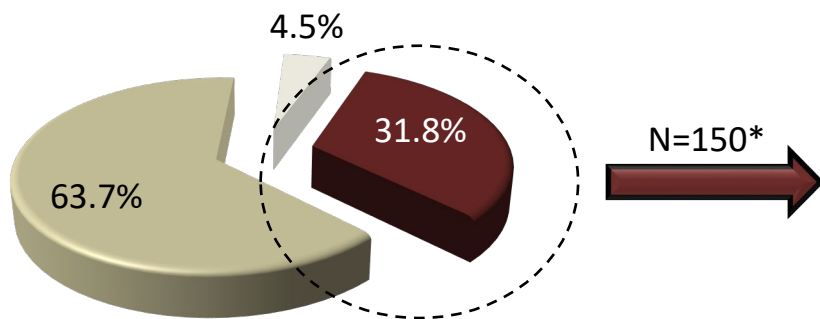
No child has hepatitis.

Data from last completed annual report of each person.

Hepatitis (ever) experienced

Experienced hepatitis

- Yes (N=150)
- No (N=300)
- Not known (N=21)






26 adults are HCV RNA positive

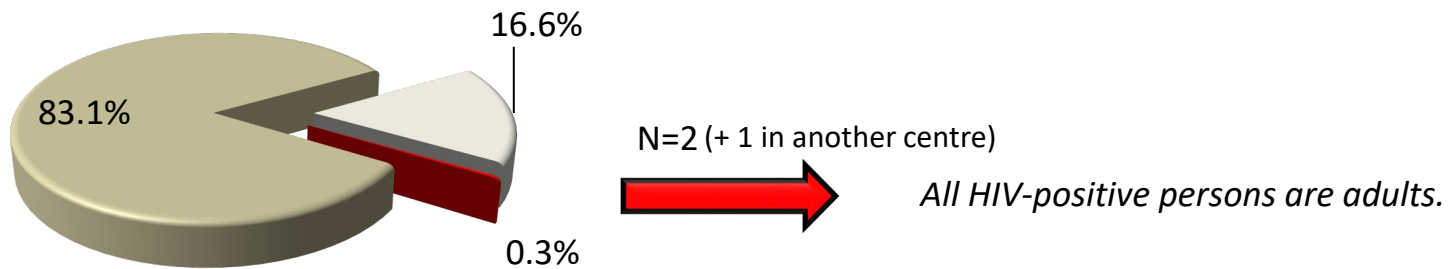
Data from last completed annual report of each person.

*Total of 218 cases of hepatitis in 150 persons. One person may have more types of hepatitis recorded.

HIV

HIV

-  Positive (N=2)
-  Negative (N=580)
-  Not known / not available (N=116)



Data from last completed annual report of each person.

Treatment outcomes and bleeding frequency

Haemophilia A

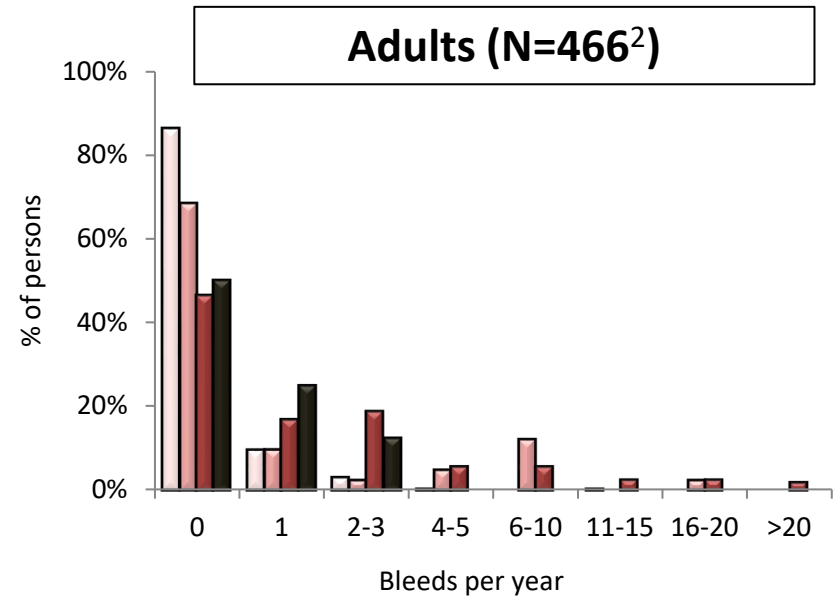
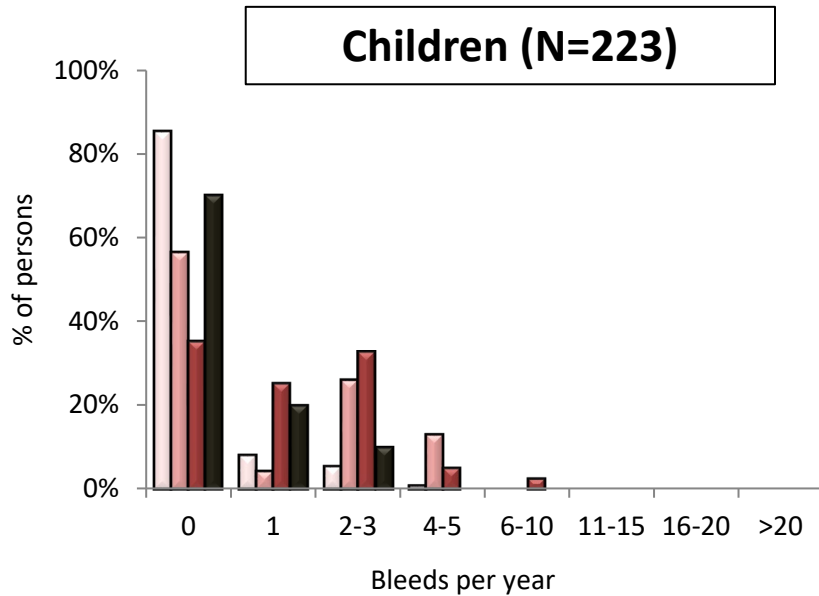


Data from year 2020 – sample size

	Valid persons		→	Persons with <u>valid</u> annual report		→	Persons <u>examined</u>		→	Persons <u>treated</u>	
	N	%		N	%		N	%		N	%
All	698	100%	→	668	95.7%	→	455	65.2%	→	347	49.7%
of them with inhibitor	18			17			18			18	
Children	227	100%	→	221	97.4%	→	185	81.5%	→	118	52.0%
of them with inhibitor	10			10			10			10	
Adults	471	100%	→	447	94.9%	→	270	57.3%	→	229	48.6%
of them with inhibitor	8			7			8			8	

Frequency of bleeding requiring treatment in 2020

¹ severity of haemophilia not known in 4 children and 4 adults



Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
110	23	80	10	N valid	258	41	160	7
0.2	1.3	1.4	0.4	Mean	0.2	1.8	2.7	0.6
0 (0–5)	0 (0–5)	1 (0–7)	0 (0–2)	Median (min – max)	0 (0–12)	0 (0–20)	1 (0–44)	0 (0–2)
94 (85.5%)	13 (56.5%)	28 (35%)	7 (70%)	N (%) with no bleed	223 (86.4%)	28 (68.3%)	74 (46.3%)	4 (50%)

* without inhibitor

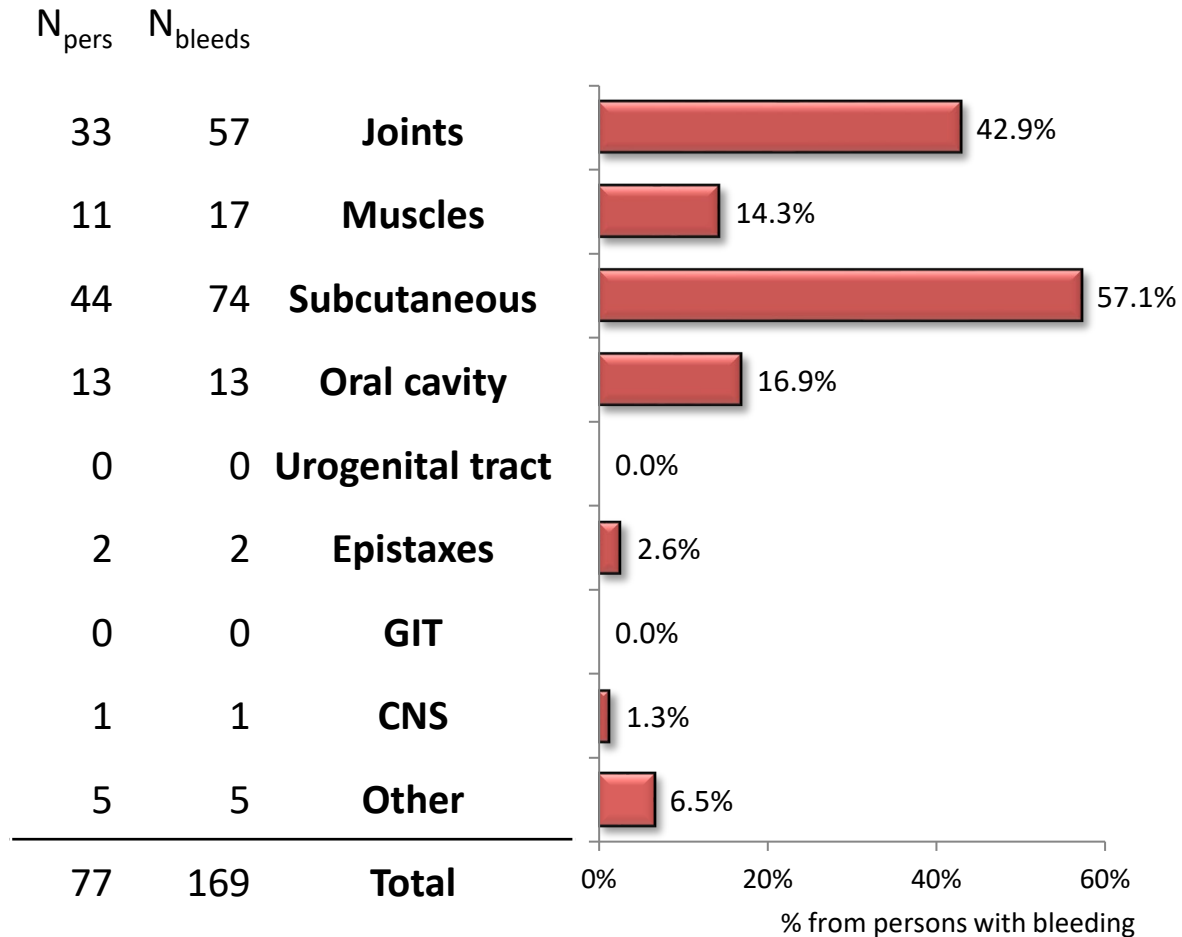
² Frequency of bleeding is missing in 1 adult (inhibitor).

Location of bleeds in 2020

81 (35.7%) children experienced bleeding requiring treatment at least once in year; 169 bleeds were recorded in total, 13 bleeds required hospitalization.

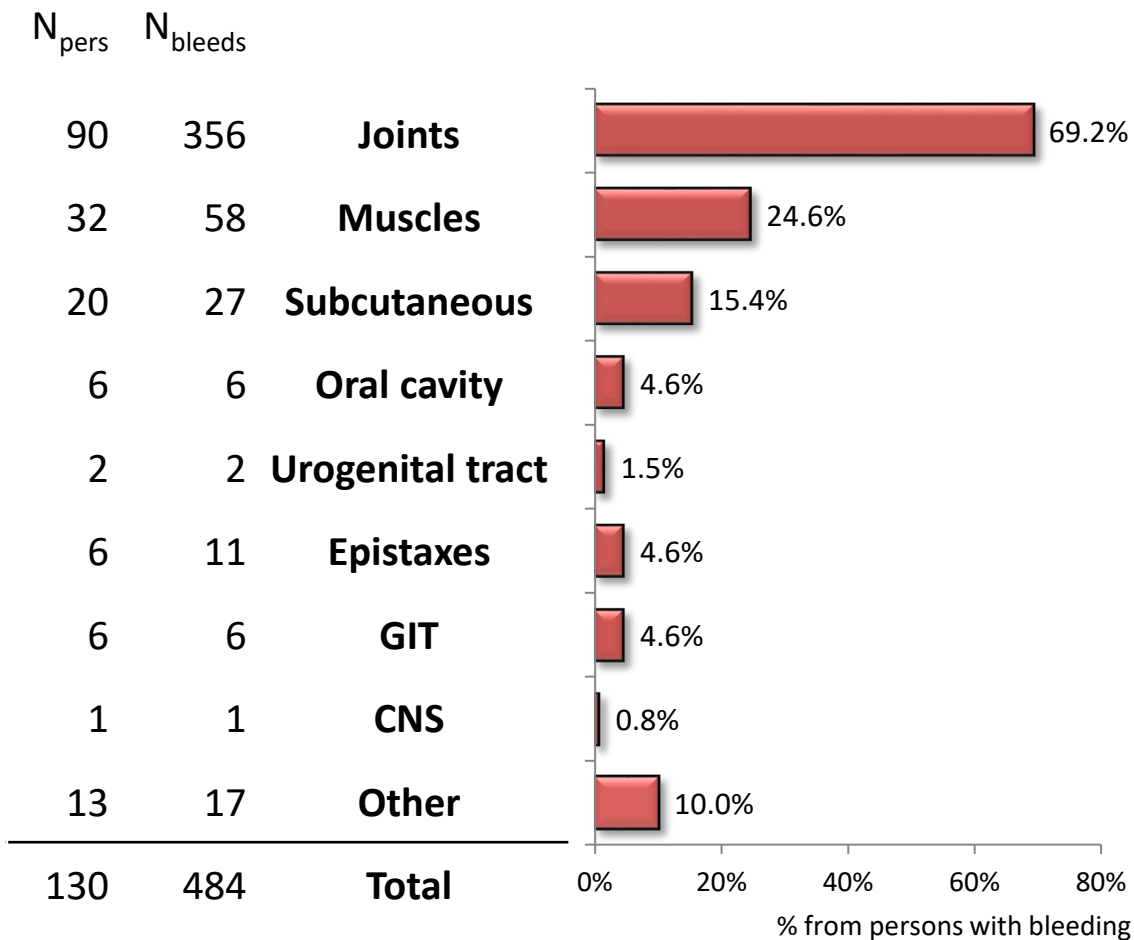
77 of these 81 children have recorded location of their bleeds. Localization is not known in 4 children.

146 (64.3%) children recorded no bleed during year 2020.



Location of bleeds in 2020

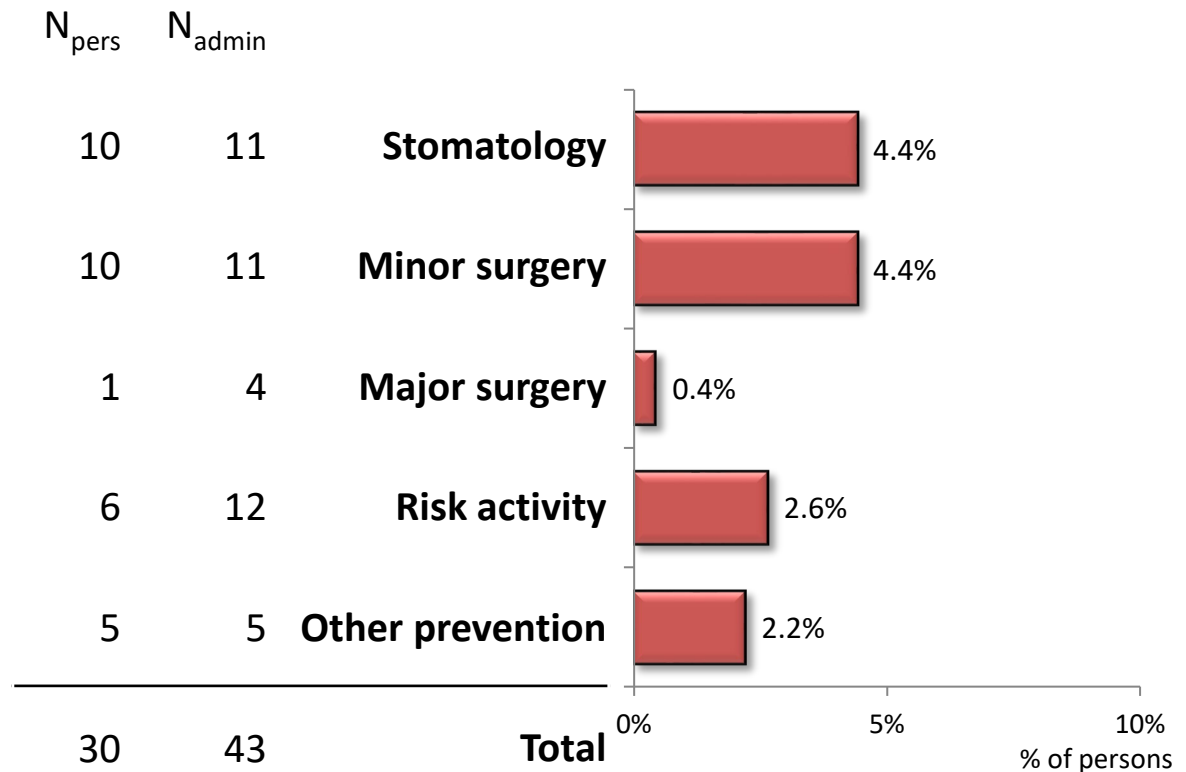
137 (29.1%) adults experienced bleeding requiring treatment at least once in year; 484 bleeds were recorded in total, 22 bleeds required hospitalization. 130 of these 137 adults have recorded location of their bleeds. Localization is not known in 7 adults. 333 (70.9%) adults have recorded no bleed during year 2020.



¹Frequency of bleeding is missing in 1 adult.

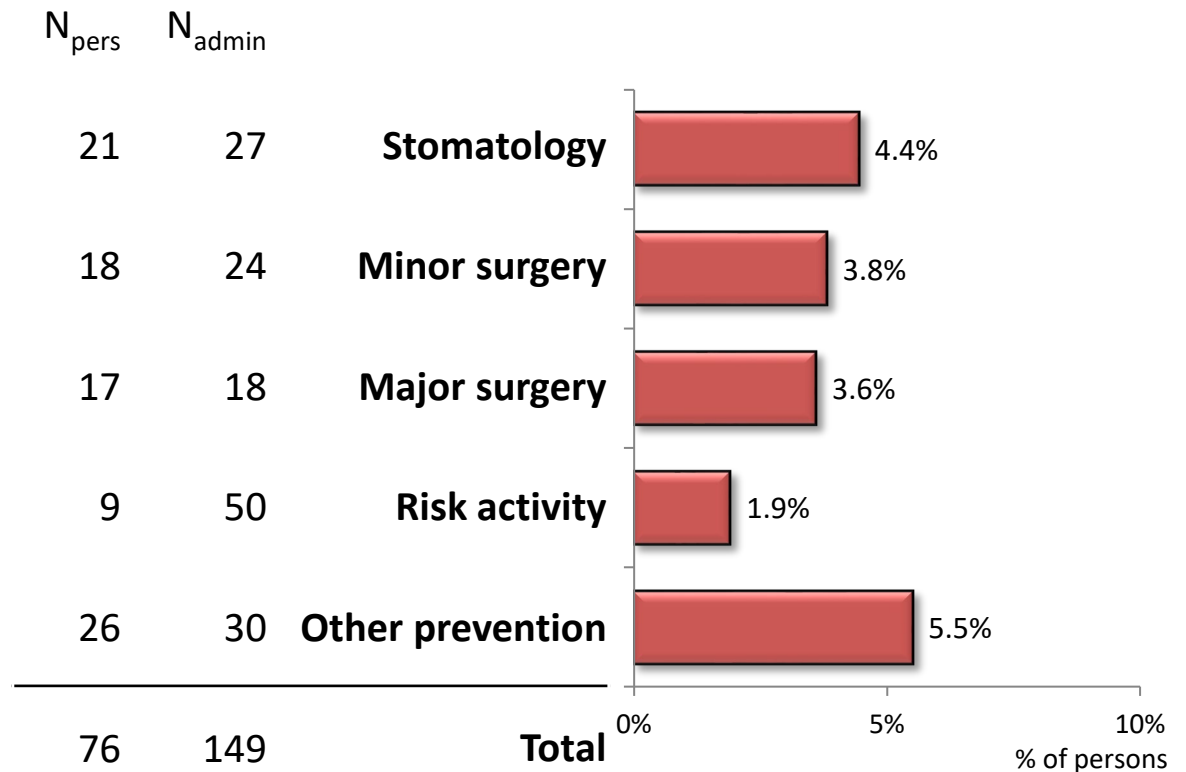
Preventive administration in 2020

30 (13.2%) children were given factor to prevent bleeding during/before risk situation.
43 preventive administrations were recorded in total.



Preventive administration in 2020

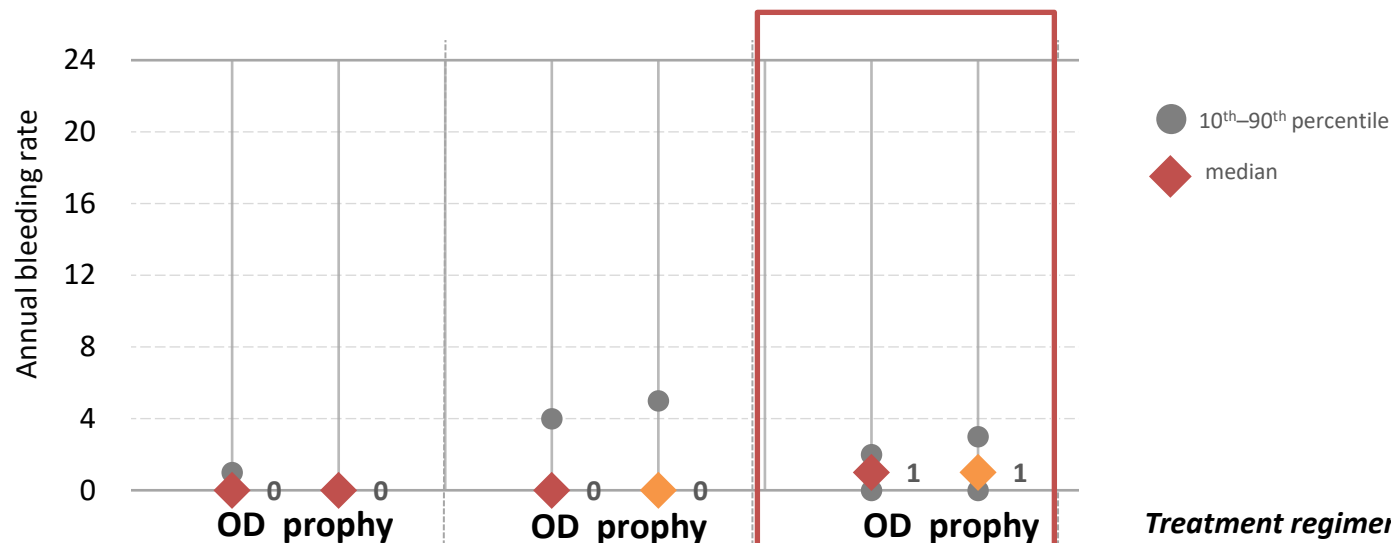
76 (16.1%) persons were given factor to prevent bleeding during/before risk situation.
149 preventive administrations were recorded in total.



ABR according to treatment regimen Haemophilia A without inhibitor



Annual bleeding rate according to treatment regimen



Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	110	0	15	8	4	76
Mean	0.2		1.5	1.0	1.0	1.4
Median (min – max)	0 (0 – 5)		0 (0 – 4)	0 (0 – 5)	1 (0 – 2)	1 (0 – 7)
Total no of recorded bleeds	27		22	8	4	109
children on permanent prophylaxis	0 (0%)		8 (34.8%)		76 (95%)	
% of factor (FVIII) consumed by children on permanent prophylaxis	-		96.1%		100.0%	

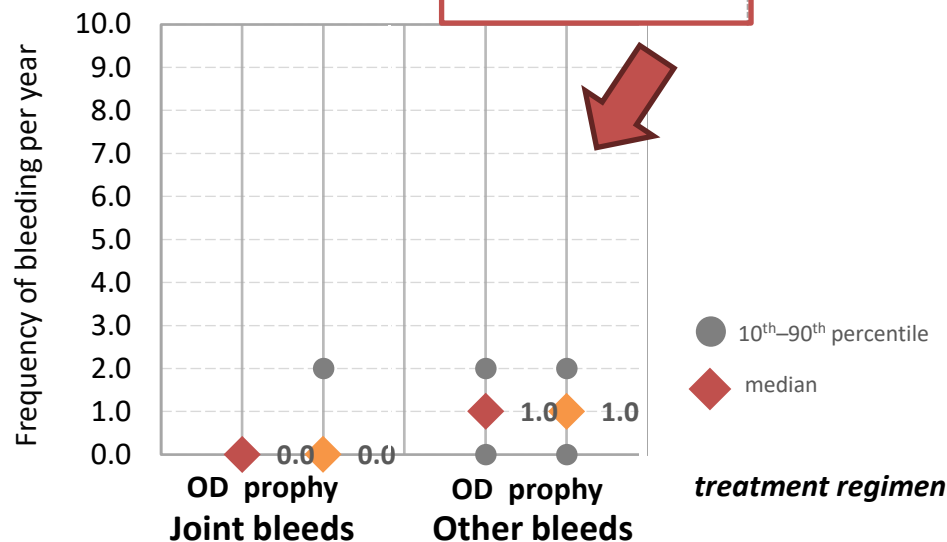
Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

* without inhibitor; missing severity in 4 children

Joint and other bleeds according to treatment regimen

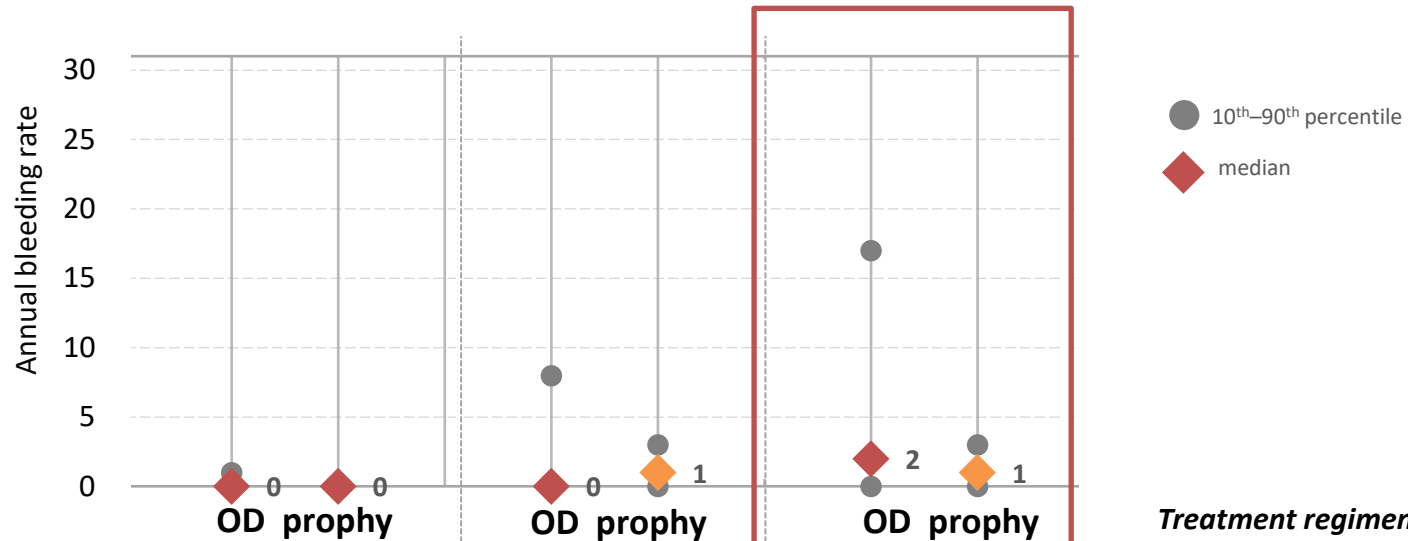
Frequency of bleeding	Mild*		Moderate*		Severe*	
	OD	prophy	OD	prophy	OD	prophy
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	107	0	15	8	4	76
JOINT BLEEDS						
Mean	0.0		0.4	0.5	0.0	0.6
Median (range)	0 (0-2)		0 (0-3)	0 (0-4)	0 (0-0)	0 (0-5)
Total no of recorded bleeds	5		6	4	0	42
OTHER BLEEDS						
Mean	0.2		1.1	0.5	1.0	0.9
Median (range)	0 (0-5)		0 (0-4)	0 (0-2)	1 (0-2)	1 (0-4)
Total no of recorded bleeds	18		16	4	4	67

* without inhibitor; missing severity in 4 children; missing location of their bleeds in 4 children (one of them is inhibitor)



Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

Annual bleeding rate according to treatment regimen



Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	258	0	38	3	46	114
Mean	0.2		1.8	1.3	5.3	1.6
Median (min – max)	0 (0 – 12)		0 (0 – 20)	1 (0 – 3)	2 (0 – 29)	1 (0 – 44)
Total no of recorded bleeds	60		69	4	245	180
adults on permanent prophylaxis	0 (0%)		3 (7.3%)		114 (71.3%)	
% of factor (FVIII) consumed by adults on permanent prophylaxis	-		57.9%		94.6%	

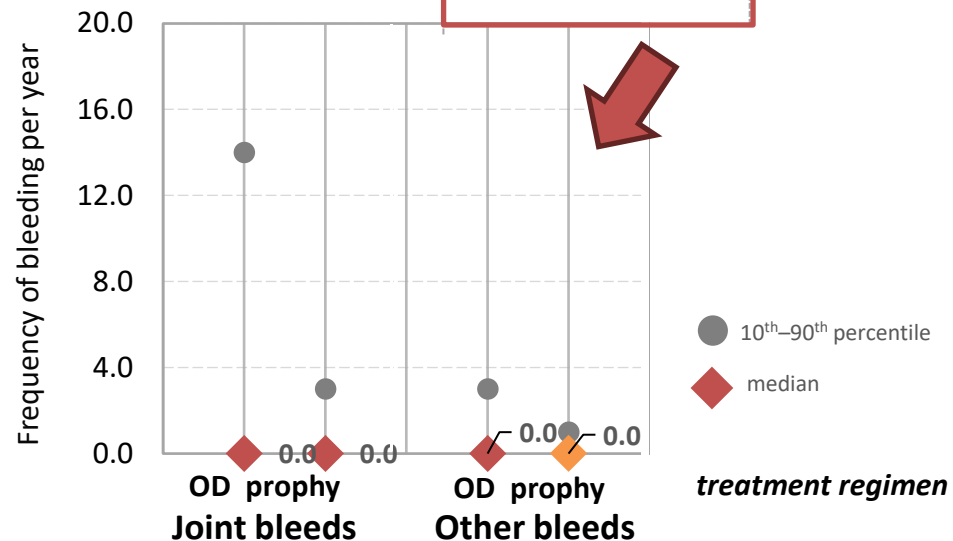
Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

* without inhibitor; missing severity in 4 adults; missing frequency of bleeding in one adult (inhibitor)

Joint and other bleeds according to treatment regimen

Frequency of bleeding	Mild*		Moderate*		Severe*	
	OD	prophy	OD	prophy	OD	prophy
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	257	0	37	3	43	112
JOINT BLEEDS						
Mean	0.1		1.2	0.3	3.7	1.2
Median (range)	0 (0-2)		0 (0-9)	0 (0-1)	0 (0-24)	0 (0-44)
Total no of recorded bleeds	13		43	1	159	137
OTHER BLEEDS						
Mean	0.1		0.2	1.0	1.2	0.3
Median (range)	0 (0-5)		0 (0-2)	0 (0-3)	0 (0-11)	0 (0-5)
Total no of recorded bleeds	34		6	3	52	32

* without inhibitor; missing severity in 4 adults; missing frequency of bleeding in one adult (inhibitor); missing location of bleeds in 7 adults



Treatment regimen:
OD = on demand and/or temporary
prophylaxis
prophy = permanent prophylaxis

ABR according to treatment regimen and age

Adults
Haem A
N=459*

* without inhibitor; missing severity in 4 adults ; missing frequency of bleeding in one adult (inhibitor)

Frequency of bleeding	Mild*		Moderate*		Severe*		
	OD	Prophy	OD	Prophy	OD	Prophy	
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	Adults (haem A) born <u>before 1990</u> N=339
N valid	194	0	22	2	40	81	
Mean	0.2		2.0	1.5	6.1	1.8	
Median (min – max)	0 (0 – 5)		0 (0 – 20)	1.5 (0 – 3)	3 (0 – 29)	1 (0 – 44)	
Total no of recorded bleeds	36		45	3	243	145	
adults on permanent prophylaxis	0 (0%)		2 (8.3%)		81 (66.9%)		
% of factor (FVIII) consumed by adults on permanent prophylaxis	-		49.9%		92.9%		
Frequency of bleeding	Mild*		Moderate*		Severe*		
	OD	Prophy	OD	Prophy	OD	Prophy	
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	Adults (haem A) born in <u>1990 or later</u> N=120
N valid	64	0	16	1	6	33	
Mean	0.4		1.5	1.0	0.3	1.1	
Median (min – max)	0 (0 – 12)		0 (0 – 8)	1 (1 – 1)	0 (0 – 2)	1 (0 – 6)	
Total no of recorded bleeds	24		24	1	2	35	
adults on permanent prophylaxis	0 (0%)		1 (5.9%)		33 (84.6%)		
% of factor (FVIII) consumed by adults on permanent prophylaxis	-		73.6%		98.8%		

Joint and other bleeds according to treatment regimen and age

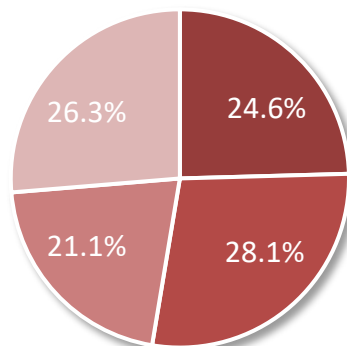
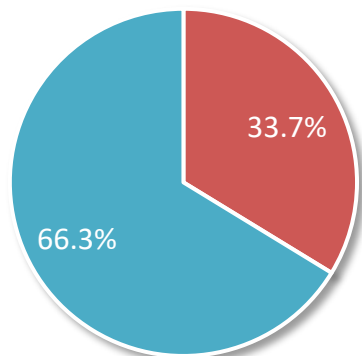
* without inhibitor; missing severity in 4 adults; missing frequency of bleeding in one adult (inhibitor); missing location of bleeds in 7 adults

Frequency of bleeding	Mild*		Moderate*		Severe*		Adults (haem A) born <u>before 1990</u> N=332
Treatment regimen	OD	prophy	OD	prophy	OD	prophy	
N valid	193	0	21	2	37	79	
JOINT BLEEDS							
Mean	0.0		1.1		4.3		Adults (haem A) born <u>in 1990 or later</u> N=120
Median (range)	0 (0-2)		0 (0-9)		1 (0-24)		
Total no of recorded bleeds	8		23		159		
OTHER BLEEDS							
Mean	0.1		0.1		1.4		
Median (range)	0 (0-5)		0 (0-2)		0 (0-11)		
Total no of recorded bleeds	27		2		50		

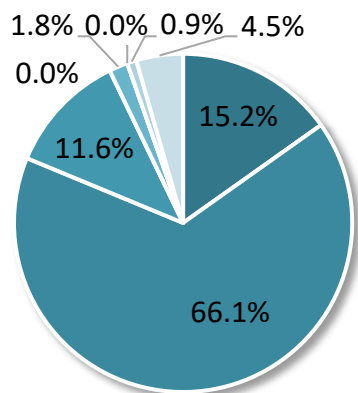
Frequency of bleeding	Mild*		Moderate*		Severe*		Adults (haem A) born <u>in 1990 or later</u> N=120
Treatment regimen	OD	prophy	OD	prophy	OD	prophy	
N valid	64	0	16	1	6	33	
JOINT BLEEDS							
Mean	0.1		1.3		0.0		Adults (haem A) born <u>in 1990 or later</u> N=120
Median (range)	0 (0-1)		0 (0-8)		0 (0-0)		
Total no of recorded bleeds	5		20		0		
OTHER BLEEDS							
Mean	0.1		0.3		0.3		
Median (range)	0 (0-2)		0 (0-2)		0 (0-2)		
Total no of recorded bleeds	7		4		2		

Location and etiology of bleeds

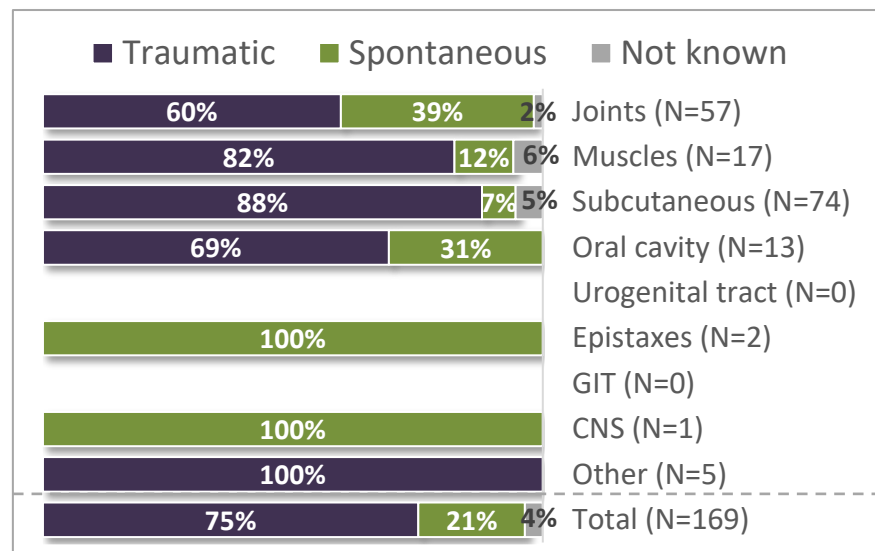
- Joints (N=57)
- Other (N=112)



- Knee (N=14)
- Ankle (N=16)
- Elbow (N=12)
- Other joint (N=15)



- Muscles (N=17)
- Subcutaneous (N=74)
- Oral cavity (N=13)
- Urogenital tract (N=0)
- Epistaxes (N=2)
- GIT (N=0)
- CNS (N=1)
- Other (N=5)



Detailed treatment of bleeds

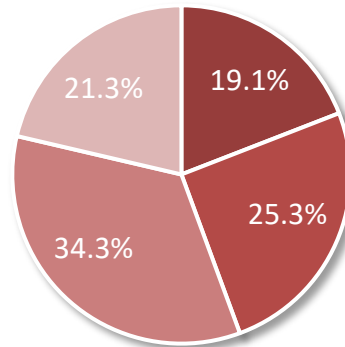
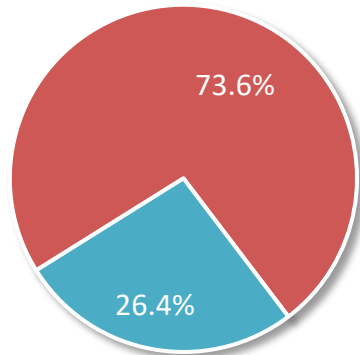
* number of bleeds

	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	57	17	74	13	0	2	0	1	5	169
FVIII consumption per bleed (IU), valid N	56	17	74	12	0	2	0	1	5	167
geometric mean	1800.5	1432.1	1196.3	1223.5		866.0		14250.0	3032.6	1455.1
median	1500.0	1500.0	1000.0	1000.0		875.0		14250.0	1500.0	1500.0
min – max	250–18000	150–18000	250–4000	500–10000		750–1000		14250–14250	500–57000	150–57000
sum	164750	49400	105250	24000		1750		14250	66000	425400
No. of doses per bleed										
geometric mean	1.6	1.9	1.3	1.4		1.0		57.0	2.5	1.5
median	1	1	1	1		1		57	2	1
min – max	1–14	1–8	1–7	1–6		1–1		57–57	1–16	1–57
Duration of therapy per bleed, days										
geometric mean	2.1	1.8	1.7	1.9		1.0		21.0	3.6	1.9
median	1	1	1	1		1		21	2	1
min – max	1–34	1–8	1–14	1–20		1–1		21–21	1–29	1–34
N (%) with hospitalization	2 (3.5%)	4 (23.5%)	4 (5.4%)	2 (15.4%)		0 (0%)		1 (100%)	0 (0%)	13 (7.7%)
N (%) with rebleeding	4 (7%)	1 (5.9%)	2 (2.7%)	0 (0%)		0 (0%)		0 (0%)	1 (20%)	8 (4.7%)

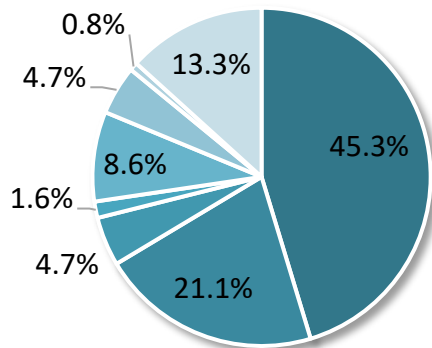
* number of bleeds

Location and etiology of bleeds

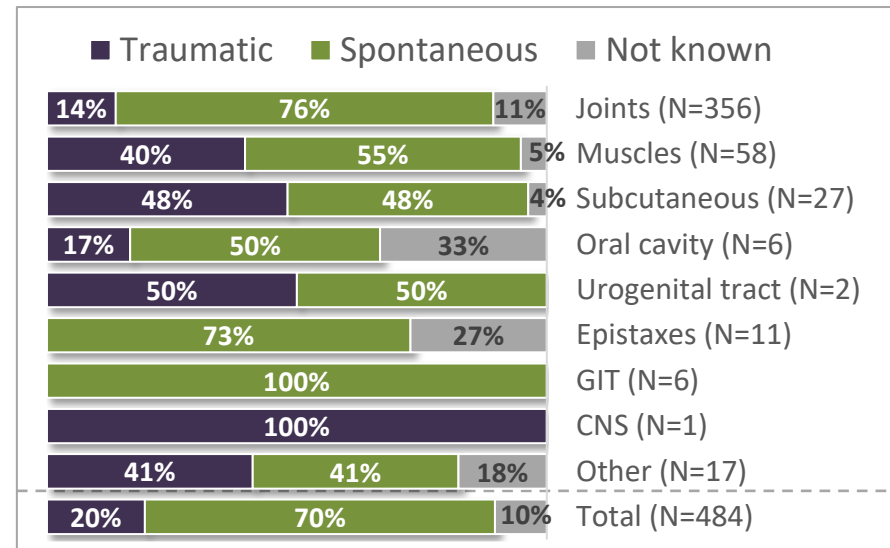
- Joints (N=356)
- Other (N=128)



- Knee (N=68)
- Ankle (N=90)
- Elbow (N=122)
- Other joint (N=76)



- Muscles (N=58)
- Subcutaneous (N=27)
- Oral cavity (N=6)
- Urogenital tract (N=2)
- Epistaxes (N=11)
- GIT (N=6)
- CNS (N=1)
- Other (N=17)



Adults
Haem A
N=484*

Detailed treatment of bleeds

* number of bleeds

	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	356	58	27	6	2	11	6	1	17	484
FVIII consumption per bleed (IU), valid N	355	57	27	6	2	11	6	1	17	482
geometric mean	2182.2	3115.7	2790.9	4185.5	11683.3	4582.2	16880.1	26000.0	3663.3	2502.1
median	2000.0	3000.0	3000.0	4500.0	13250.0	3000.0	23000.0	26000.0	3000.0	2000.0
min – max	500–115000	500–54000	500–20000	1500–16000	7000–19500	1000–48500	3000–51000	26000–26000	500–49000	500–115000
sum	1315000	373000	113500	36500	26500	110000	140000	26000	177000	2317500
No. of doses per bleed										
geometric mean	1.7	2.6	1.5	2.8	9.2	3.3	7.7	13.0	2.5	1.9
median	1	2	1	3	10	2	9	13	1	1
min – max	1–200 ¹	1–32	1–7	1–8	7–12	1–50	1–30	13–13	1–23	1–200
Duration of therapy per bleed, days										
geometric mean	1.5	2.1	1.6	2.5	5.5	2.6	4.2	7.0	1.9	1.7
median	1	1	1	3	6	1	7	7	1	1
min – max	1–250 ¹	1–33	1–6	1–15	5–6	1–34	1–10	7–7	1–16	1–250
N (%) with hospitalization	6 (1.7%)	4 (6.9%)	0 (0%)	0 (0%)	1 (50%)	0 (0%)	5 (83.3%)	1 (100%)	5 (29.4%)	22 (4.5%)
N (%) with rebleeding	23 (6.5%)	1 (1.7%)	1 (3.7%)	1 (16.7%)	0 (0%)	2 (18.2%)	1 (16.7%)	0 (0%)	1 (5.9%)	30 (6.2%)

¹ 200 doses and 250 days of therapy was used for complicated meniscus fracture

ABR according to centres Haemophilia A (PWHA)

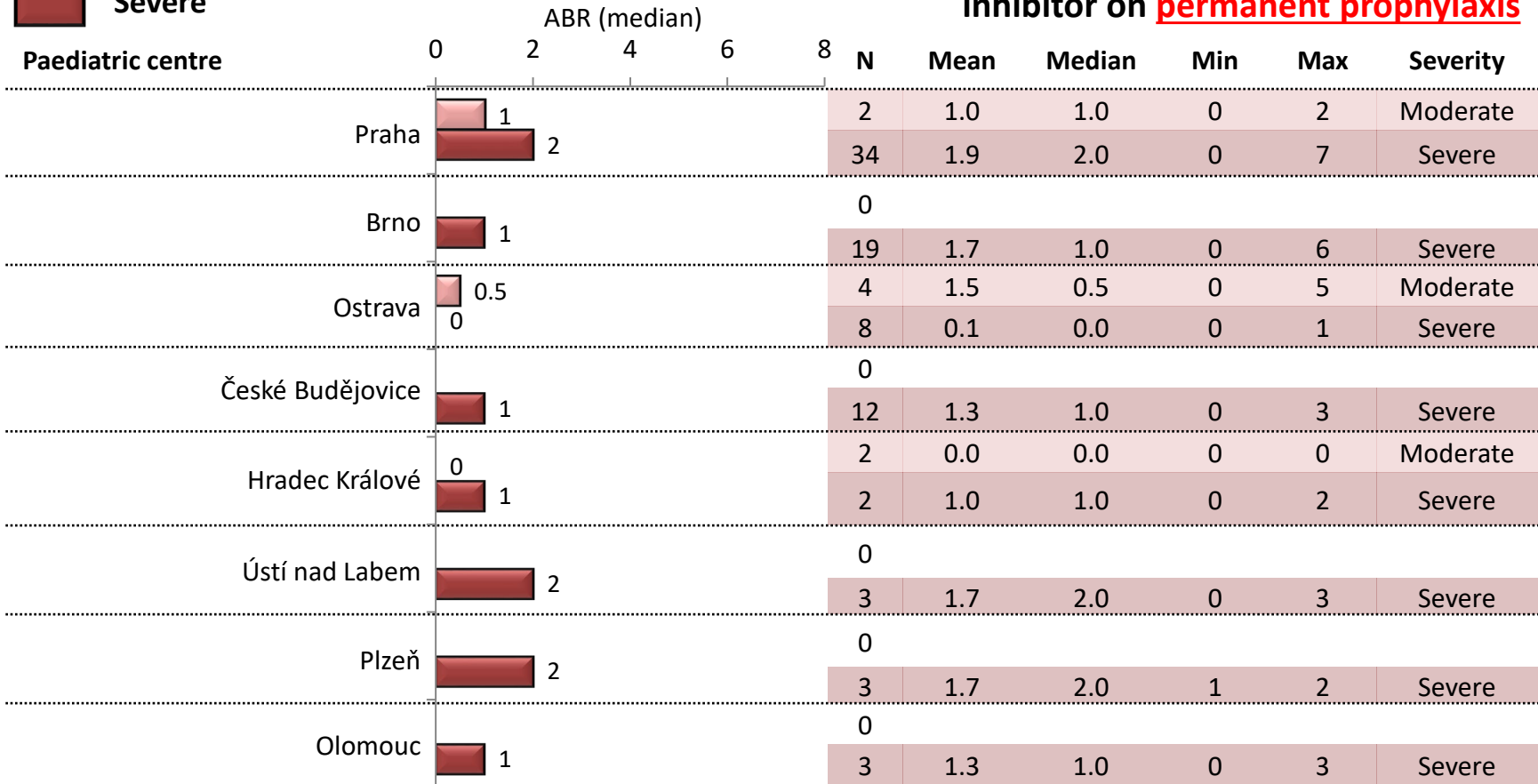


Annual bleeding rate on permanent prophylaxis

HaemA on prophylaxis
Paed. centres
N=92



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

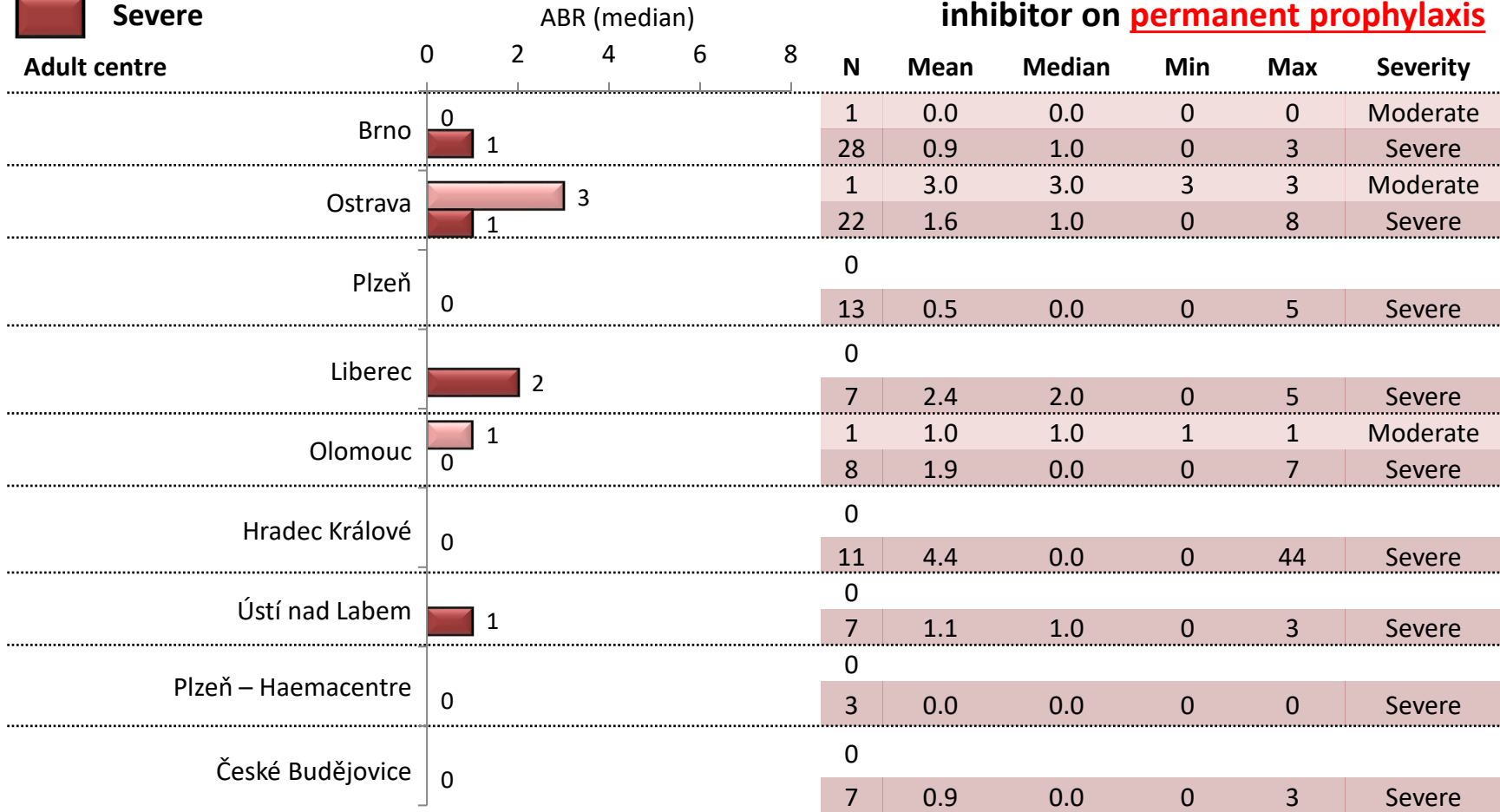


Annual bleeding rate on permanent prophylaxis

HaemA on prophylaxis
Adult centres
N=109

 Moderate
 Severe

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

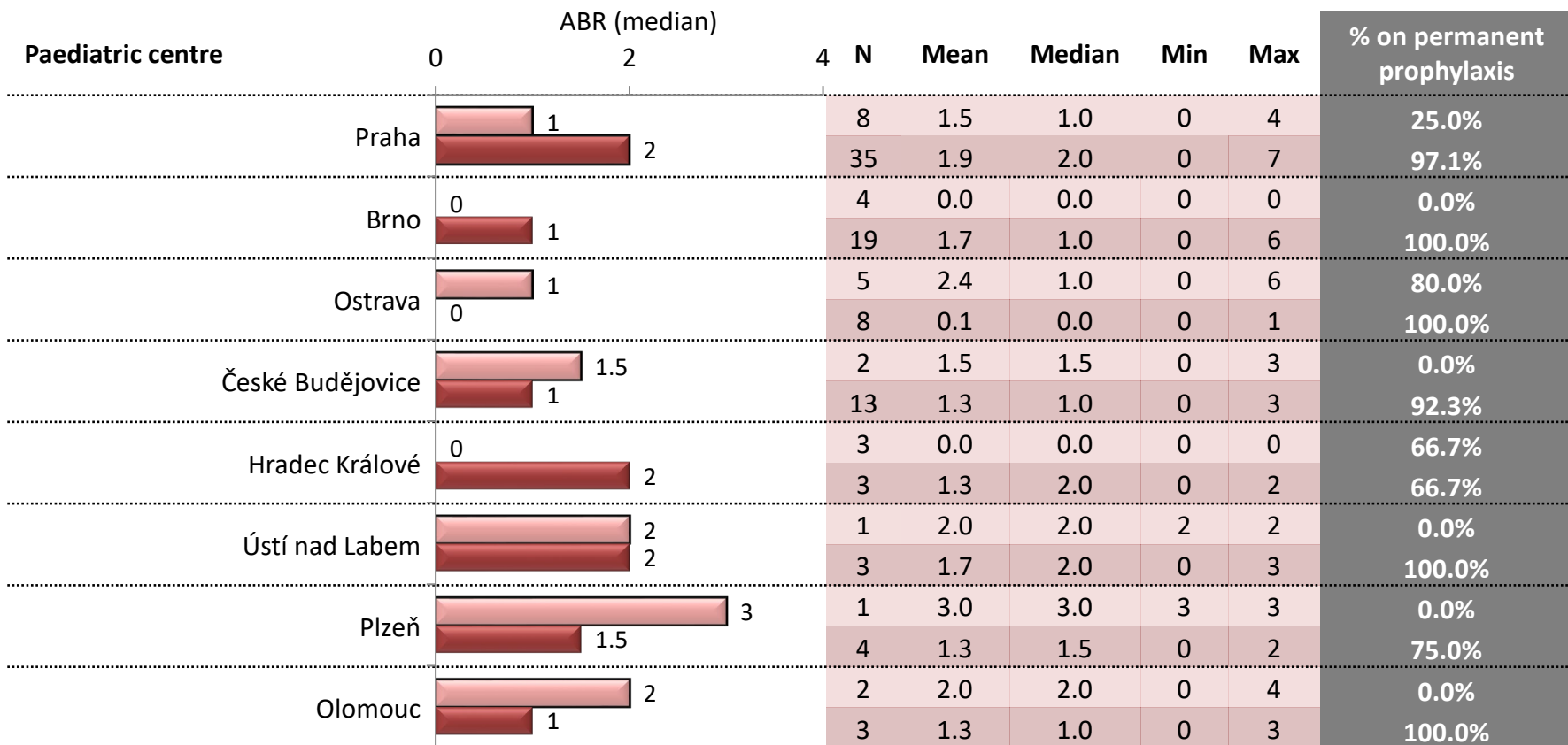


Annual bleeding rate regardless prophylaxis

HaemA
Paed. centres
N=114



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



Annual bleeding rate regardless prophylaxis

HaemA
Adult centres
N=190*

* missing ABR in 1 adult



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

Adult centre	ABR (median)					N	Mean	Median	Min	Max	% on permanent prophylaxis
	0	2	4	6	8						
Brno	0.0					14	0.4	0.0	0	4	7.1%
	1.0					38	0.9	1.0	0	3	73.7%
Ostrava	3.0					5	4.0	3.0	0	9	20.0%
	1.0					25	1.6	1.0	0	8	88.0%
Plzeň	0.0					3	6.7	0.0	0	20	0.0%
	0.0					20	2.0	0.0	0	12	65.0%
Liberec	0.0					1	0.0	0.0	0	0	0.0%
	4.0					11	6.0	4.0	0	29	63.6%
Olomouc	4.0					2	4.0	4.0	1	7	50.0%
	2.5					20	6.4	2.5	0	25	40.0%
Hradec Králové	0.0					5	0.4	0.0	0	1	0.0%
	0.0					17	4.2	0.0	0	44	64.7%
Ústí nad Labem	0.0					3	0.0	0.0	0	0	0.0%
	1.5					8	1.9	1.5	0	7	87.5%
Plzeň – Haemacentre	4.0					1	4.0	4.0	4	4	0.0%
	0.0					3	0.0	0.0	0	0	100.0%
České Budějovice	0.0					4	2.0	0.0	0	8	0.0%
	0.0					10	1.0	0.0	0	4	70.0%

Prophylactic regimens and treatment outcomes

HaemA
Paed. centres
N=114

Paediatric centre	Severity	Total N	PERMANENT PROPHYLAXIS								ON-DEMAND / TEMPORARY PROPHY		
			% of patients	N	Dosing of prophylaxis (IU/kg per week)				ABR		N	ABR	
					Mean	Median	Min	Max	Mean	Median		Mean	Median
Praha	Moderate	8	25.0%	2	49.9	49.9	44.9	54.8	59.9	59.9	6	6.0	7.0
	Severe	35	97.1%	34	78.7	72.9	31.3	120.2	0.0	0.0	1	1.0	3.0
Brno	Moderate	4	0.0%	0							4	4.0	2.0
	Severe	19	100.0%	19	80.7	86.7	22.1	125.0	0.0	0.0	0		
Ostrava	Moderate	5	80.0%	4	44.9	43.5	41.3	51.2	0.0	0.0	1	1.0	0.0
	Severe	8	100.0%	8	80.3	76.4	51.3	119.3	0.0	0.0	0		
Č. Budějovice	Moderate	2	0.0%	0							2	2.0	1.0
	Severe	13	92.3%	12	67.9	65.8	36.1	117.2	0.0	0.0	1	1.0	0.0
Hradec Králové	Moderate	3	66.7%	2	47.2	47.2	27.2	67.2	0.0	0.0	1	1.0	1.0
	Severe	3	66.7%	2	95.9	95.9	95.2	96.5	0.0	0.0	1	1.0	0.0
Ústí nad Labem	Moderate	1	0.0%	0							1	1.0	0.0
	Severe	3	100.0%	3	83.3	62.5	46.4	141.0	0.0	0.0	0		
Plzeň	Moderate	1	0.0%	0							1	1.0	1.0
	Severe	4	75.0%	3	106.8	72.6	52.5	195.3	0.0	0.0	1	1.0	1.0
Olomouc	Moderate	2	0.0%	0							2	2.0	1.0
	Severe	3	100.0%	3	29.1	29.1	14.6	43.5	0.0	0.0	0		

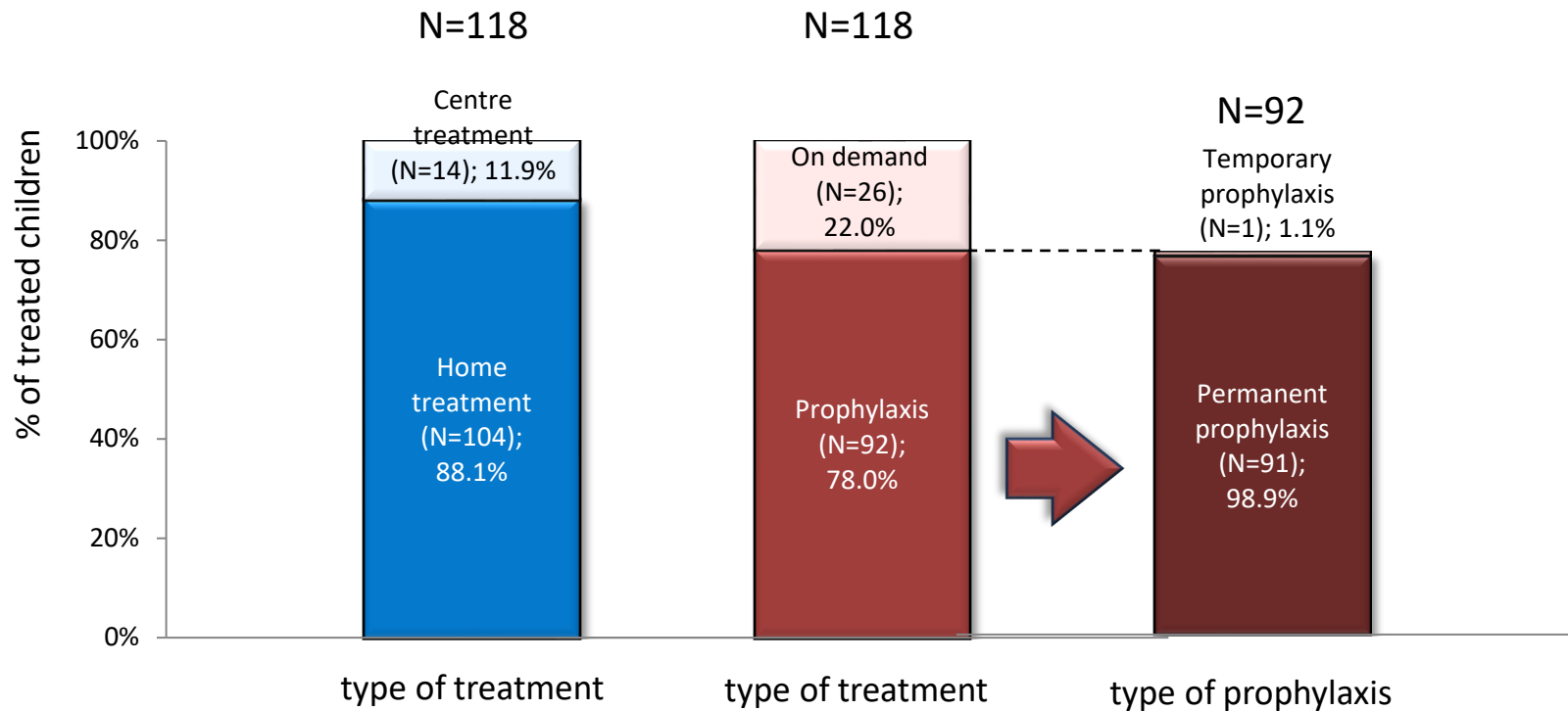
Prophylactic regimens and treatment outcomes

HaemA
Adult centres
N=190*

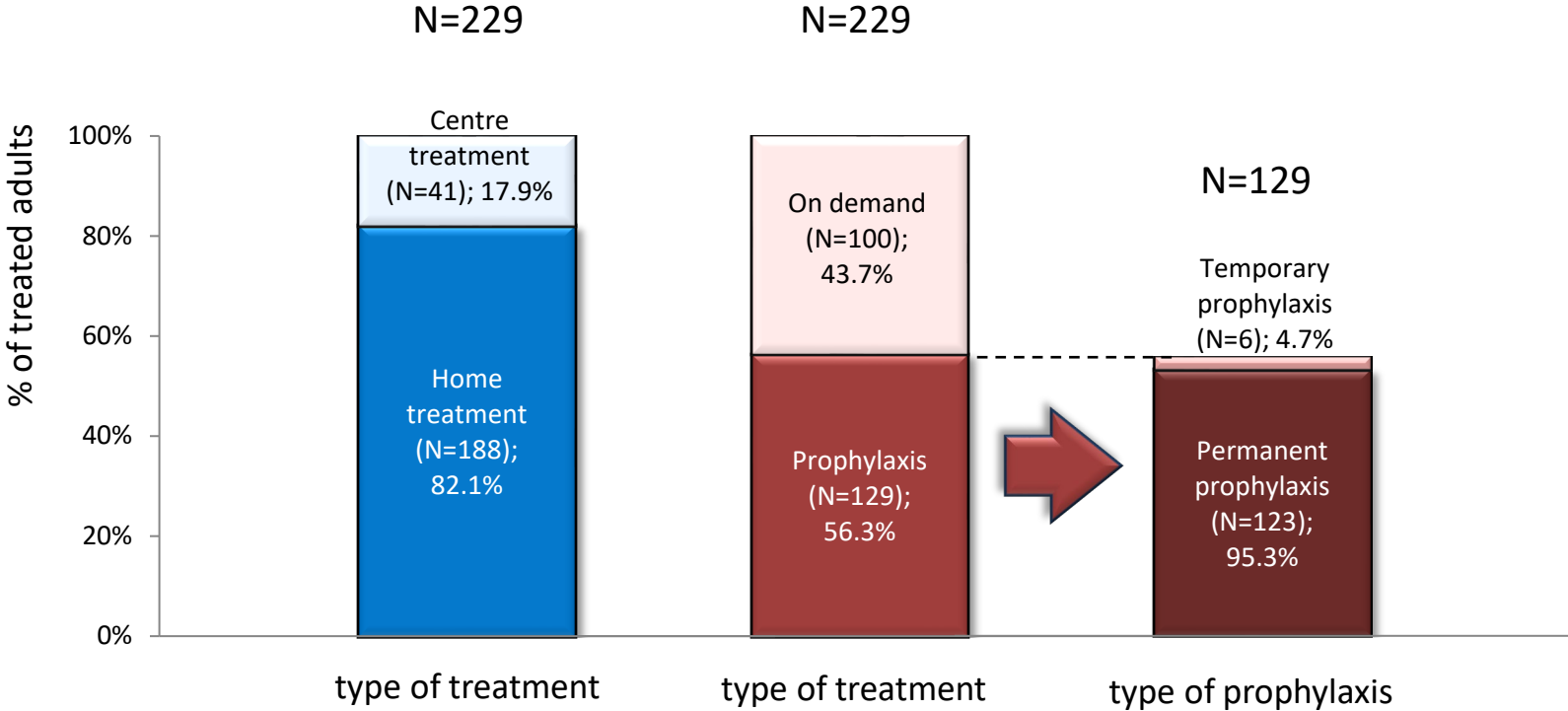
* missing ABR in 1 adult

Adult centre	Severity	Total N	PERMANENT PROPHYLAXIS									ON-DEMAND / TEMPORARY PROPHY				
			% of patients	N	Dosing of prophylaxis (IU/kg per week)				ABR*		Age	N	ABR		Age	
					Mean	Median	Min	Max	Mean	Median	Median		Mean	Median	Median	
Brno	Moderate	14	7.1%	1	59.1	59.1	59.1	59.1	59.1	0.0	0.0	31	13	0.4	0.0	46
	Severe	38	73.7%	28	64.3	63.0	23.1	121.6	0.9	1.0	39	10	0.9	0.0	45	
Ostrava	Moderate	5	20.0%	1	97.7	97.7	97.7	97.7	3.0	3.0	68	4	4.3	4.0	55	
	Severe	25	88.0%	22	61.9	58.3	30.8	106.2	1.6	1.0	41	3	1.7	1.0	67	
Plzeň	Moderate	3	0.0%	0								3	6.7	0.0	44	
	Severe	20	65.0%	13	52.4	47.7	21.9	100.0	0.5	0.0	43	7	4.9	0.0	63	
Liberec	Moderate	1	0.0%	0								1	0.0	0.0	56	
	Severe	11	63.6%	7	63.8	55.8	51.1	92.9	2.4	2.0	36	4	12.3	9.0	66	
Olomouc	Moderate	2	50.0%	1	76.9	76.9	76.9	76.9	1.0	1.0	21	1	7.0	7.0	25	
	Severe	20	40.0%	8	47.5	50.5	27.8	68.2	1.9	0.0	40	12	9.4	7.5	48	
Hradec Králové	Moderate	5	0.0%	0								5	0.4	0.0	25	
	Severe	17	64.7%	11	66.0	66.8	31.8	101.6	4.4	0.0	34	6	4.0	1.0	38	
Ústí n. Labem	Moderate	3	0.0%	0								3	0.0	0.0	23	
	Severe	8	87.5%	7	50.5	50.0	21.4	76.9	1.1	1.0	36	1	7.0	7.0	53	
Plzeň - Haemacentre	Moderate	1	0.0%	0								1	4.0	4.0	51	
	Severe	3	100.0%	3	55.8	60.2	30.3	76.9	0.0	0.0	47	0				
Č. Budějovice	Moderate	4	0.0%	0								4	2.0	0.0	71	
	Severe	10	70.0%	7	50.1	52.6	32.1	80.0	0.9	0.0	52	3	1.3	0.0	49	

Type of treatment (subgroup of treated patients)



Type of treatment (subgroup of treated patients)



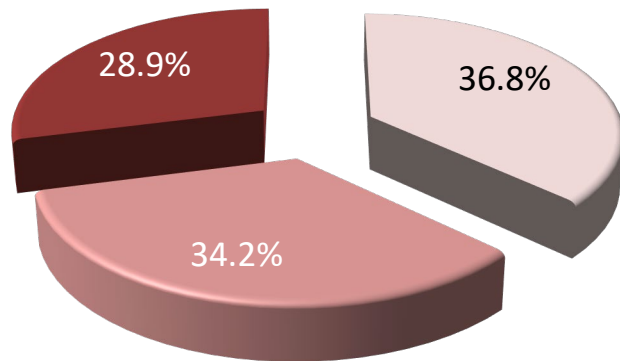
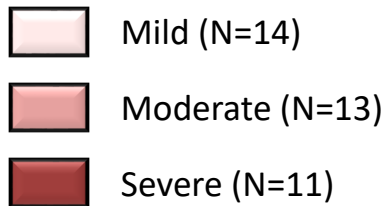
Demographic characteristics

Haemophilia B

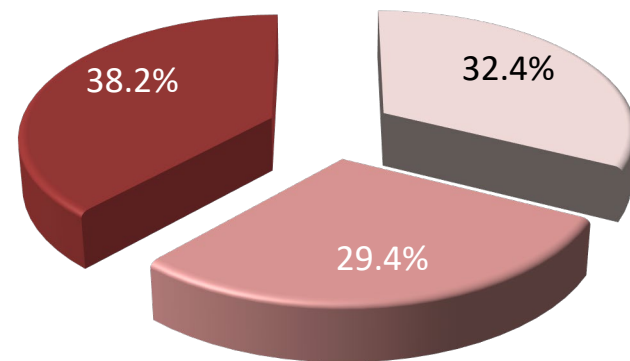
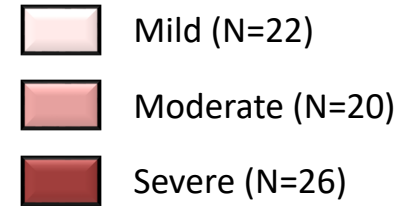


Severity of haemophilia B

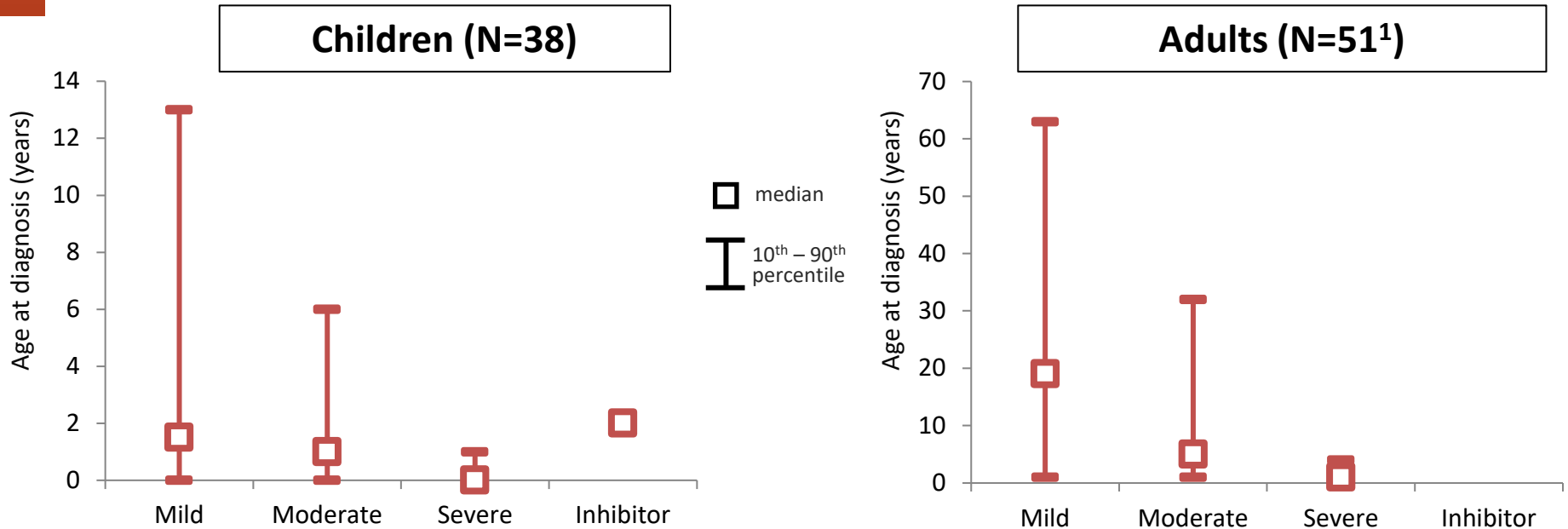
Children (N=38)



Adults (N=68)



Age at diagnosis according to severity of haemophilia B

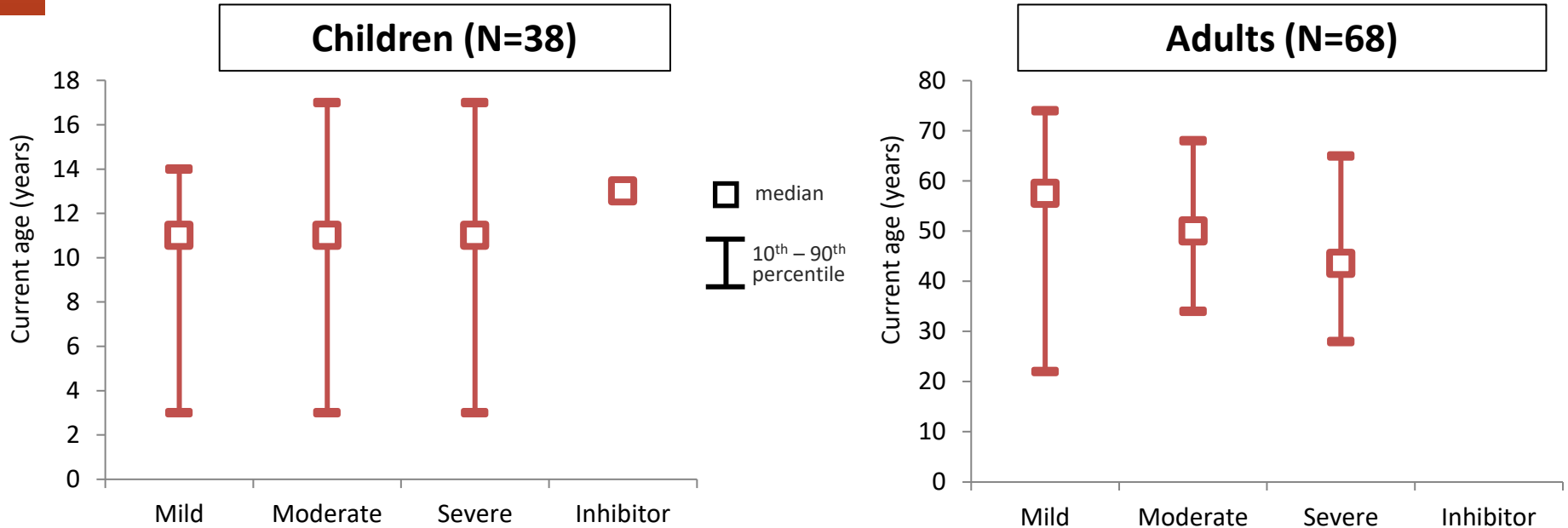


Mild*	Moderate*	Severe*	Inhibitor ⁺	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor ⁺
14	13	11	1	N valid	15	15	21	0
3.9	2.3	0.5	2.0	Mean	29.9	10.0	1.7	
1.5 (0 – 15)	1 (0 – 8)	0 (0 – 2)	2 (2 – 2)	Median (min – max)	19 (0 – 67)	5 (0 – 61)	1 (0 – 8)	

¹ Missing information on year of diagnosis in 17 adults.

* including persons with inhibitor
+ in 2020

Actual age according to severity of haemophilia B



Mild*	Moderate*	Severe*	Inhibitor ⁺	Current age ⁺⁺ (years)	Mild*	Moderate*	Severe*	Inhibitor ⁺
14	13	11	1	N valid	22	20	26	0
9.5	10.8	9.9	13.0	Mean	50.7	50.2	44.8	
11 (2 – 16)	11 (1 – 18)	11 (1 – 18)	13 (13 – 13)	Median (min – max)	57.5 (19 – 95)	50 (21 – 71)	43.5 (25 – 69)	

* including persons with inhibitor

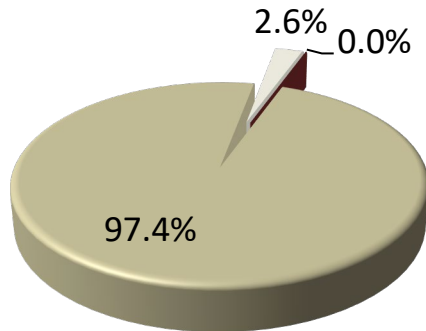
⁺ in 2020

⁺⁺ age reached in year 2020

Hepatitis (ever) experienced

Experienced hepatitis

- Yes (N=0)
- No (N=37)
- Not known (N=1)



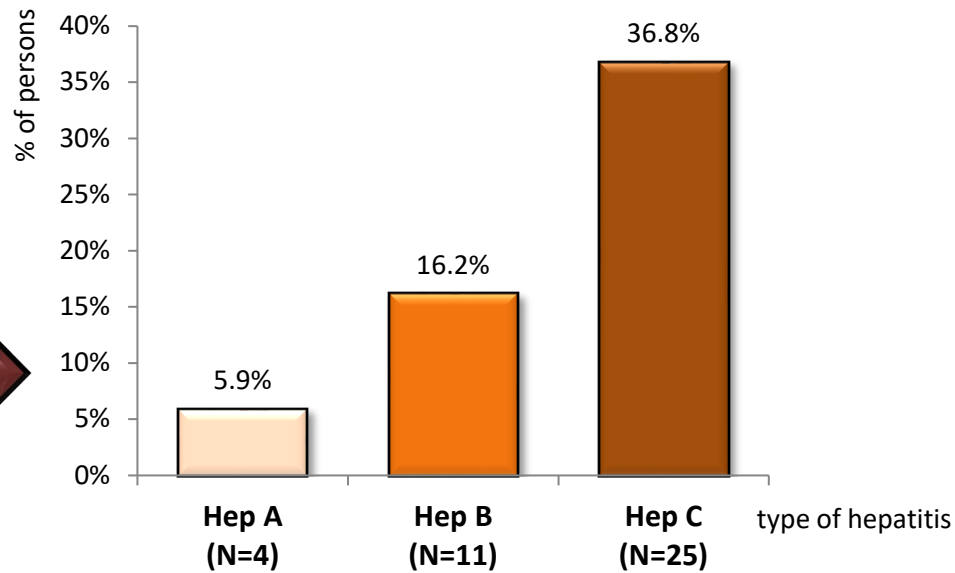
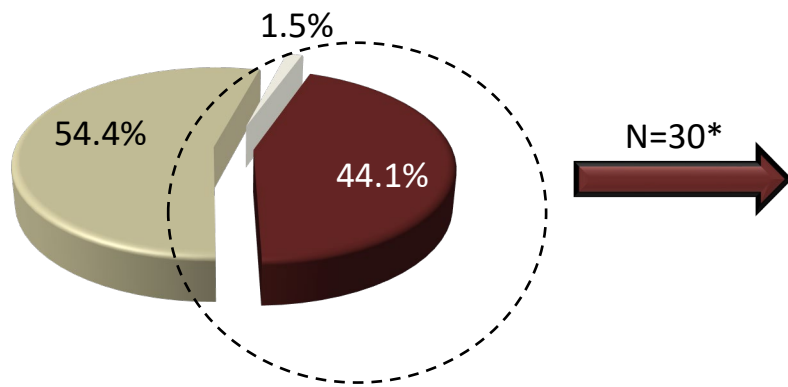
No child has hepatitis C.

Data from last completed annual report of each person.

Hepatitis (ever) experienced

Experienced hepatitis

- Yes (N=30)
- No (N=37)
- Not known (N=1)



9 adults are HCV RNA positive

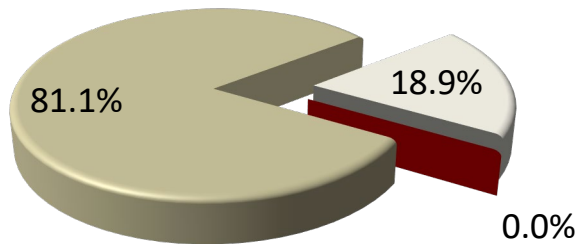
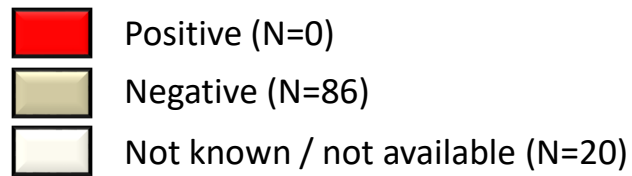
Data from last completed annual report of each person.

*Total of 40 cases of hepatitis in 30 persons. One person may have more types of hepatitis recorded.

HIV

All
Haem B
N=106

HIV



No HIV-positive person.

Data from last completed annual report of each person.

Treatment outcomes and bleeding frequency

Haemophilia B

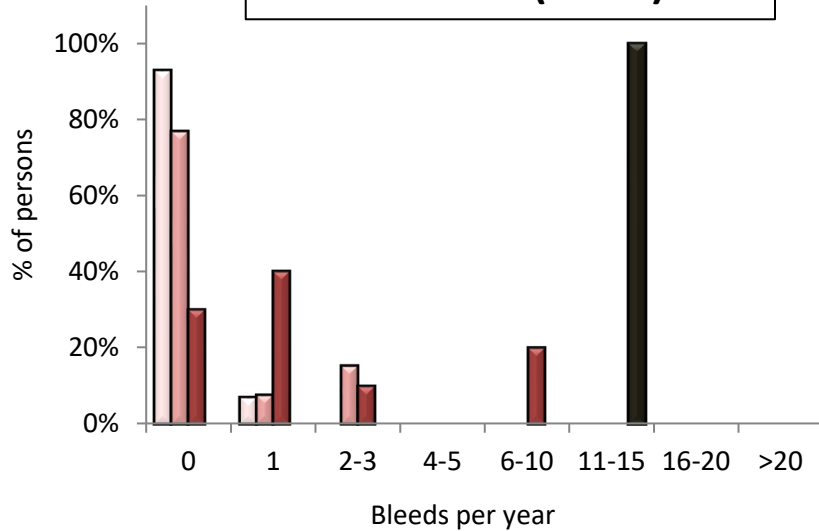
All
Haem B
N=106

Data from year 2020 – sample size

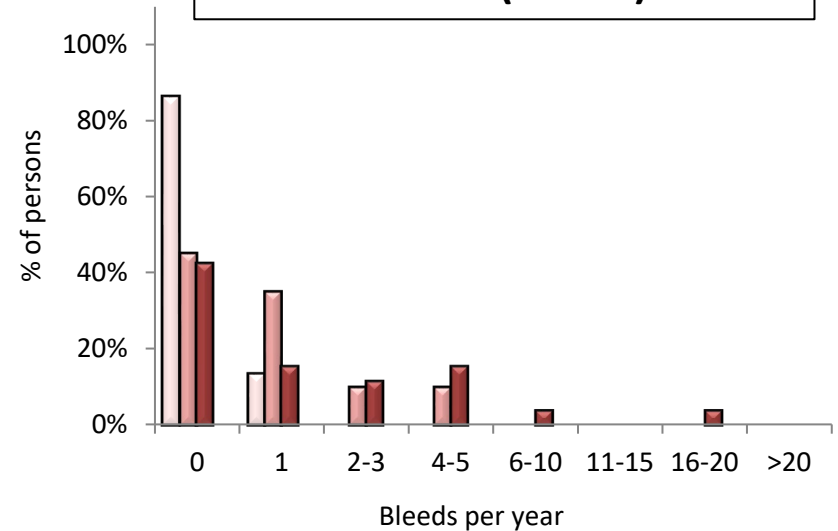
	Valid persons		→	Persons with <u>valid</u> annual report		→	Persons <u>examined</u>		→	Persons <u>treated</u>	
	N	%		N	%		N	%		N	%
All	106	100%	→	101	95.3%	→	83	78.3%	→	57	53.8%
of them with inhibitor	1			1			1			1	
Children	38	100%	→	37	97.4%	→	31	81.6%	→	16	42.1%
of them with inhibitor	1			1			1			1	
Adults	68	100%	→	64	94.1%	→	52	76.5%	→	41	60.3%
of them with inhibitor	0			0			0			0	

Frequency of bleeding requiring treatment in 2019

Children (N=38)



Adults (N=66¹)



Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
14	13	10	1	N valid	22	20	24	0
0.1	0.4	2.1	13.0	Mean	0.1	1.0	2.2	0.0
0 (0 – 1)	0 (0 – 2)	1 (0 – 9)	13 (13 – 13)	Median (min – max)	0 (0 – 1)	1 (0 – 4)	1 (0 – 16)	(–)
13 (92.9%)	10 (76.9%)	3 (30%)	0 (0%)	N (%) with no bleed	19 (86.4%)	9 (45%)	11 (42.3%)	0 (0%)

* without inhibitor

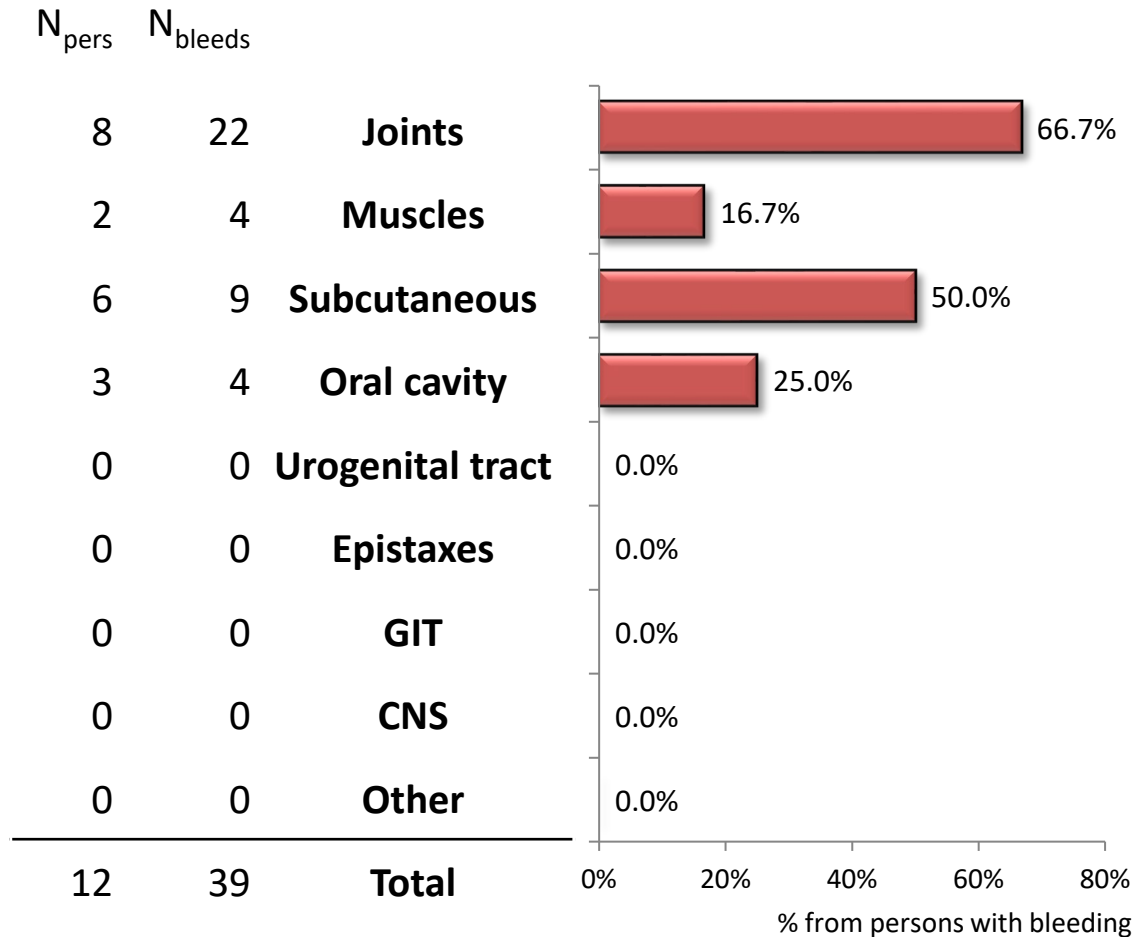
¹Frequency of bleeding is missing in 2 adults.

Location of bleeds in 2020

12 (31.6%) children experienced bleeding requiring treatment at least once in year; 39 bleeds were recorded in total, 3 bleeds required hospitalization.

All of these children have recorded location of their bleeds.

26 (68.4%) children recorded no bleed during year 2020.

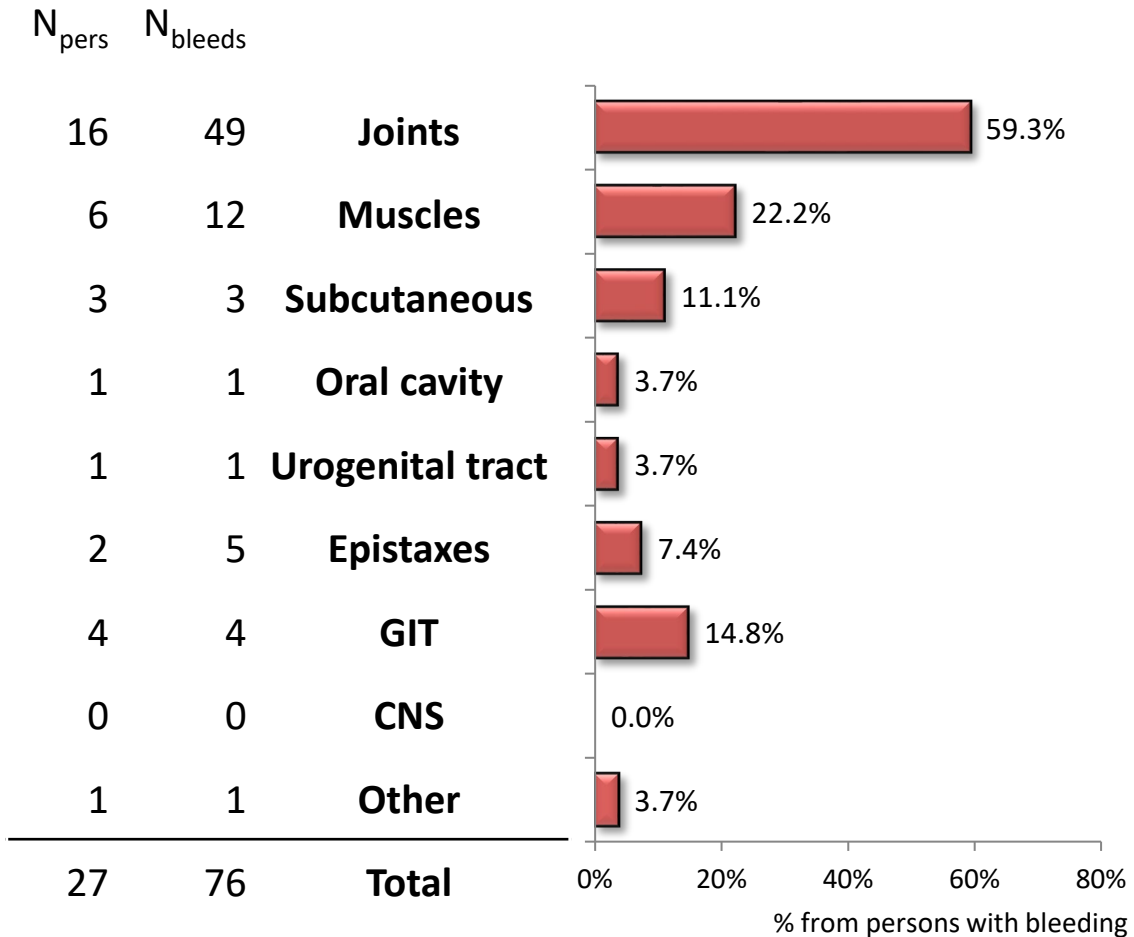


Location of bleeds in 2020

27 (40.9%) adults experienced bleeding requiring treatment at least once in year; 76 bleeds were recorded in total, 4 bleeds required hospitalization.

All of these 27 adults have recorded location of their bleeds.

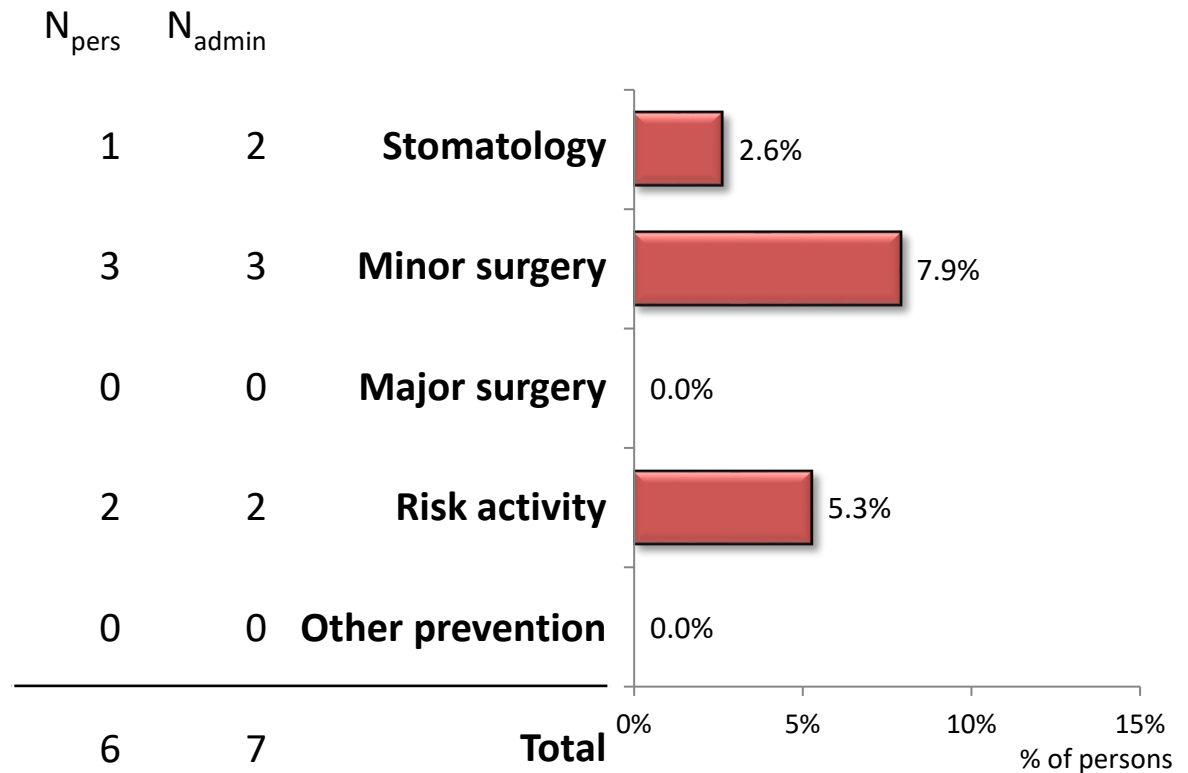
39 (59.1%) adults have recorded no bleed during year 2020.



¹Frequency of bleeding is missing in 2 adults.

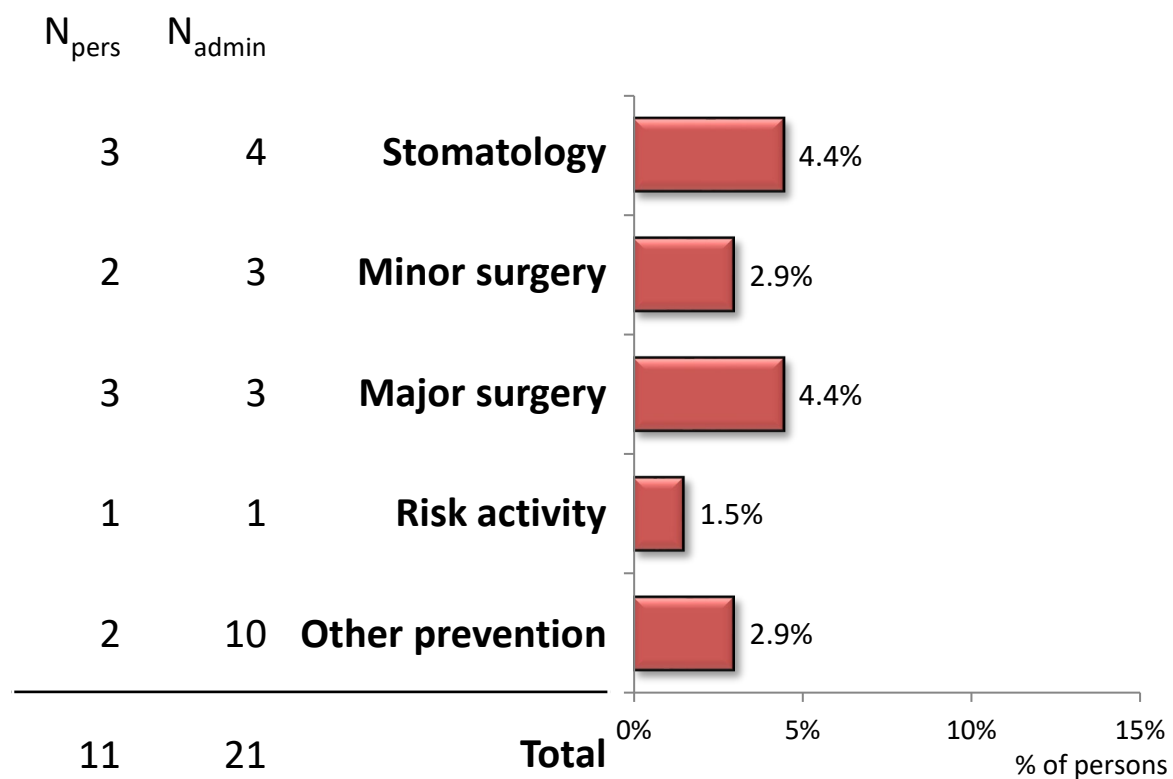
Preventive administration in 2020

6 (15.8%) children were given factor to prevent bleeding during/before risk situation.
7 preventive administrations were recorded in total.



Preventive administration in 2020

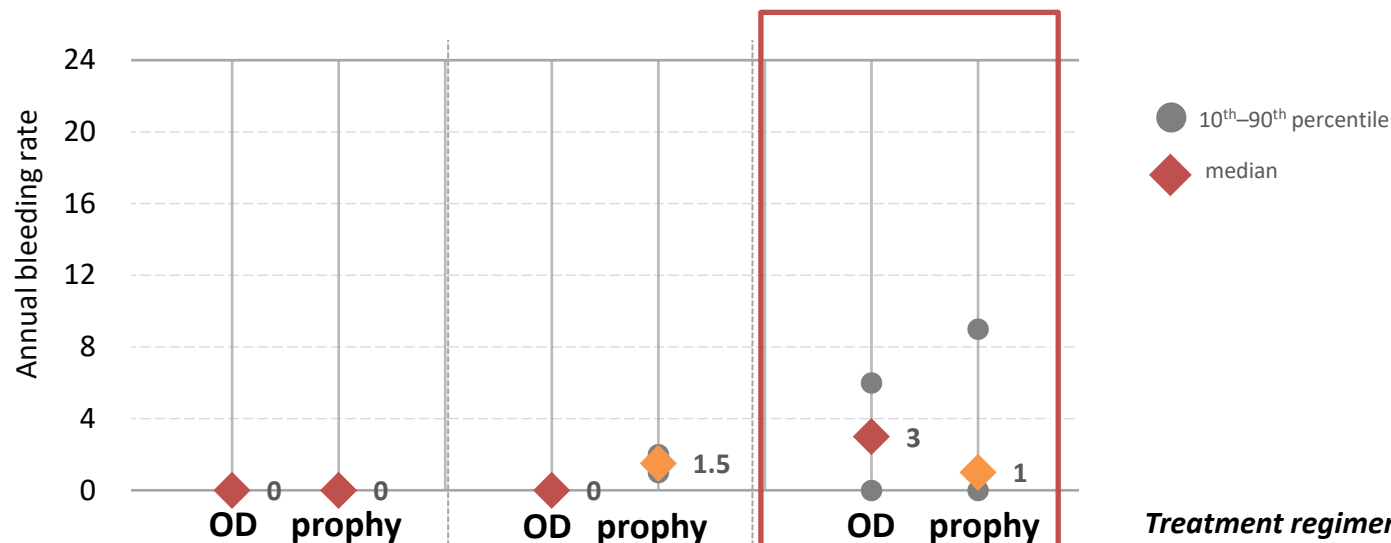
11 (16.2%) persons were given factor to prevent bleeding during/before risk situation.
21 preventive administrations were recorded in total.



ABR according to treatment regimen Haemophilia B without inhibitor



Annual bleeding rate according to treatment regimen



Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	14	0	11	2	2	8
Mean	0.1		0.2	1.5	3.0	1.9
Median (min – max)	0 (0 – 1)		0 (0 – 2)	1.5 (1 – 2)	3 (0 – 6)	1 (0 – 9)
Total no of recorded bleeds	1		2	3	6	15
children on permanent prophylaxis	0 (0%)		2 (15.4%)		8 (80%)	
% of factor (FIX) consumed by children on permanent prophylaxis	-		99.1%		76.5%	

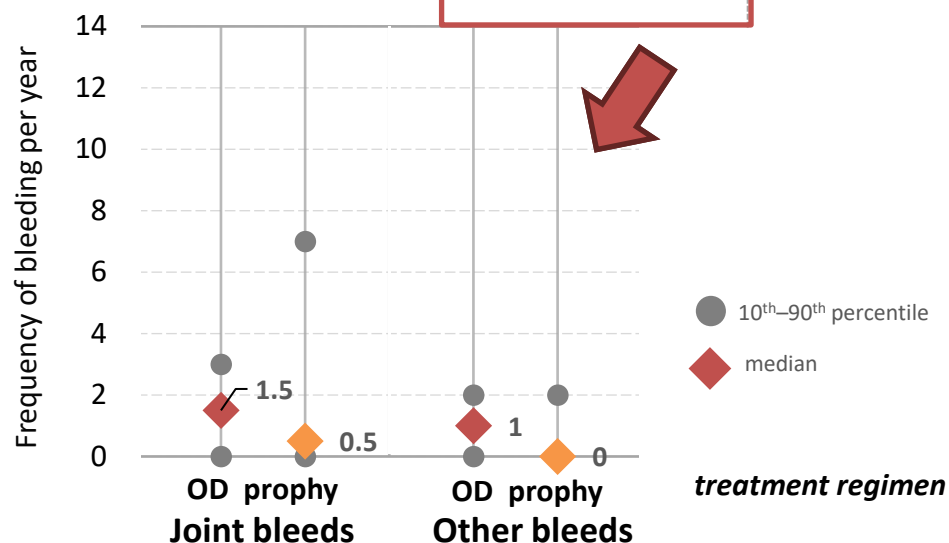
Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

* without inhibitor

Joint and other bleeds according to treatment regimen

* without inhibitor

Frequency of bleeding	Mild*		Moderate*		Severe*	
	OD	prophy	OD	prophy	OD	prophy
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	14	0	11	2	2	8
JOINT BLEEDS						
Mean	0.0	0	0.0	1.0	1.5	1.4
Median (range)	0 (0-0)	(-)	0 (0-0)	1 (1-1)	1.5 (0-3)	0.5 (0-7)
Total no of recorded bleeds	0	0	0	2	3	11
OTHER BLEEDS						
Mean	0.1	0	0.2	0.5	1.0	0.5
Median (range)	0 (0-1)	(-)	0 (0-2)	0.5 (0-1)	1 (0-2)	0 (0-2)
Total no of recorded bleeds	1	0	2.00	1	2	4

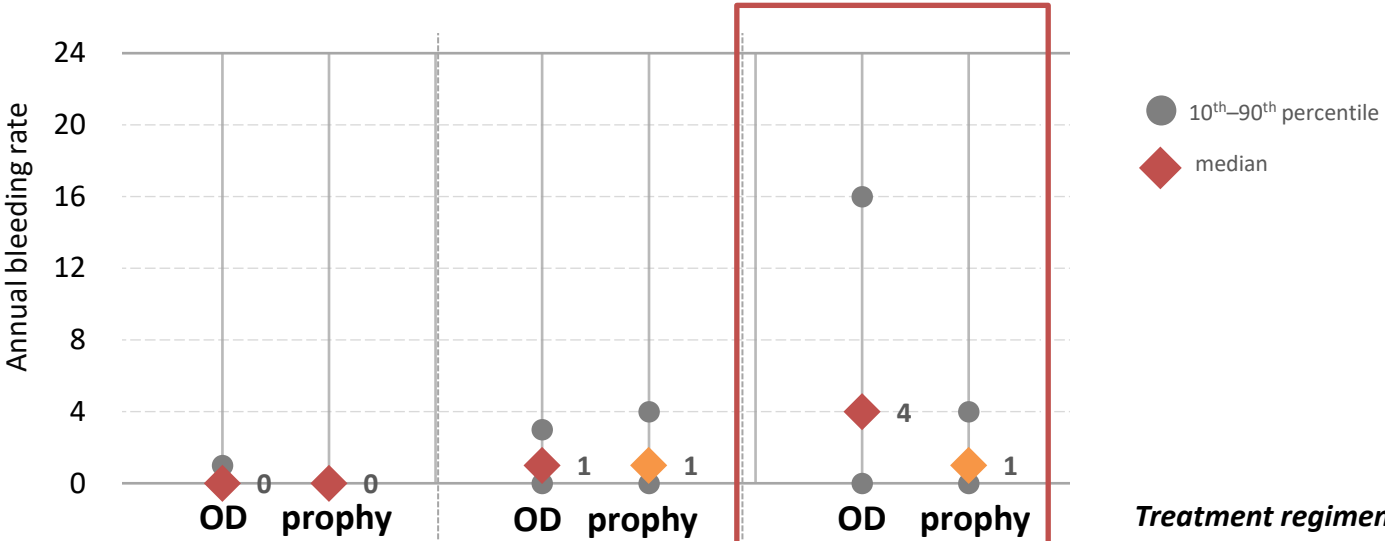


Treatment regimen:

OD = on demand and/or temporary
prophylaxis

prophy = permanent prophylaxis

Annual bleeding rate according to treatment regimen



Frequency of bleeding	Mild*		Moderate*		Severe*	
N total	22	0	17	3	7	17
Mean	0.1		0.9	1.7	4.1	1.4
Median (min – max)	0 (0 – 1)		1 (0 – 4)	1 (0 – 4)	4 (0 – 16)	1 (0 – 8)
Total no of recorded bleeds	3		15	5	29	24
adults on permanent prophylaxis	0 (0%)		3 (15%)		18 (69.2%)	
% of factor (FIX) consumed by adults on permanent prophylaxis	-		58.5%		93.0%	

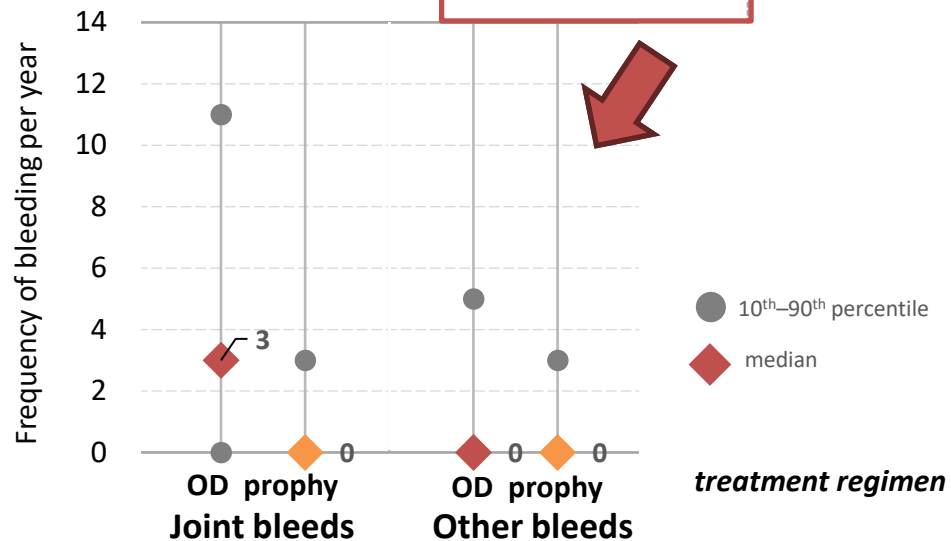
Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

* without inhibitor;
missing frequency of bleeding in 2 adults

Joint and other bleeds according to treatment regimen

Frequency of bleeding	Mild*		Moderate*		Severe*	
	OD	prophy	OD	prophy	OD	prophy
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	22	0	17	3	7	17
JOINT BLEEDS						
Mean	0.0	0	0.5	1.3	3.3	0.8
Median (range)	0 (0-0)	(-)	0 (0-4)	1 (0-3)	3 (0-11)	0 (0-8)
Total no of recorded bleeds	0	0	8	4	23	14
OTHER BLEEDS						
Mean	0.1	0	0.4	0.3	0.9	0.6
Median (range)	0 (0-1)	(-)	0 (0-3)	0 (0-1)	0 (0-5)	0 (0-3)
Total no of recorded bleeds	3	0	7	1	6	10

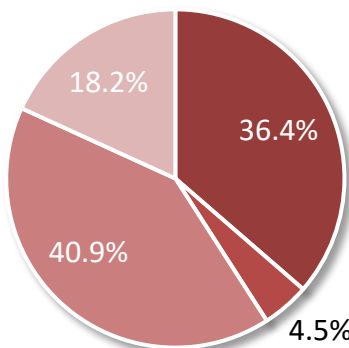
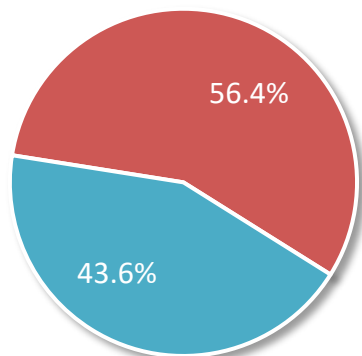
* without inhibitor; missing frequency of bleeding in 2 adults



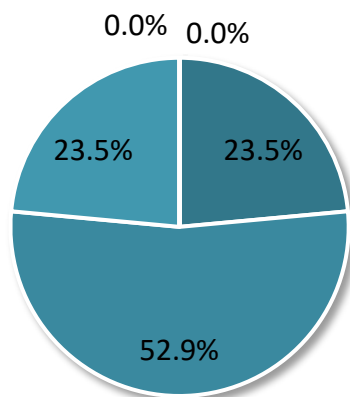
Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

Location and etiology of bleeds

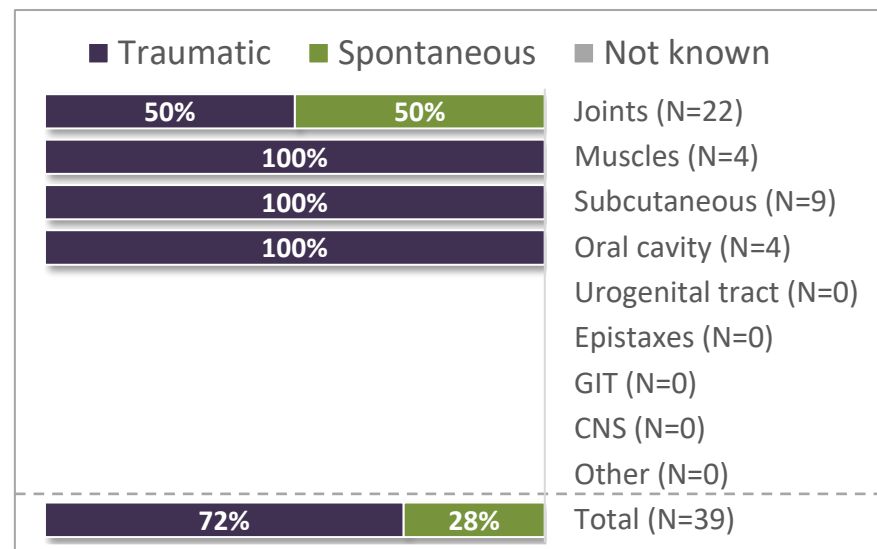
- Joints (N=22)
- Other (N=17)



- Knee (N=8)
- Ankle (N=1)
- Elbow (N=9)
- Other joint (N=4)



- Muscles (N=4)
- Subcutaneous (N=9)
- Oral cavity (N=4)
- Urogenital tract (N=0)
- Epistaxes (N=0)
- GIT (N=0)
- CNS (N=0)
- Other (N=0)



Detailed treatment of bleeds

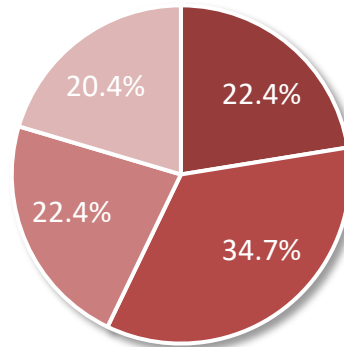
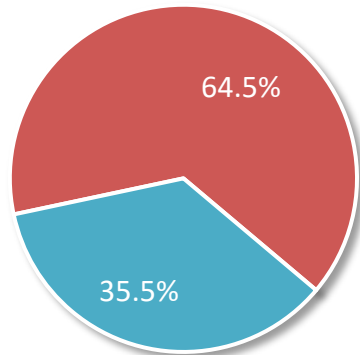
* number of bleeds

	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	22	4	9	4	0	0	0	0	0	39
FIX consumption per bleed (IU), valid N	16	1	5	4	0	0	0	0	0	26
geometric mean	2582.7	41000.0	4042.8	1170.2						2771.9
median	2500.0	41000.0	4000.0	1500.0						3000.0
min – max	500–18000	41000–41000	2000–9000	500–3000						500–41000
sum	57000	41000	23000	6500						127500
No. of doses per bleed										
geometric mean	1.4	3.5	1.5	1.5						1.6
median	1	3	1	1						1
min – max	1–4	2–8	1–7	1–5						1–8
Duration of therapy per bleed, days										
geometric mean	1.8	4.7	1.9	3.8						2.2
median	1	3	1	5						2
min – max	1–8	3–18	1–9	1–10						1–18
N (%) with hospitalization	1 (4.5%)	0 (0%)	0 (0%)	2 (50%)						3 (7.7%)
N (%) with rebleeding	1 (4.5%)	0 (0%)	0 (0%)	2 (50%)						3 (7.7%)

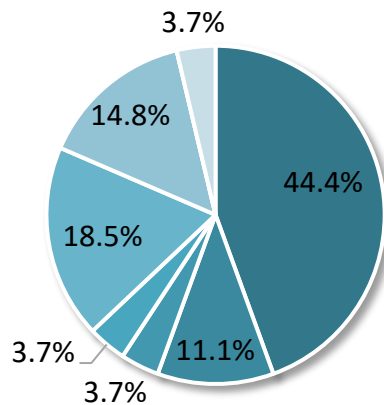
* number of bleeds

Location and etiology of bleeds

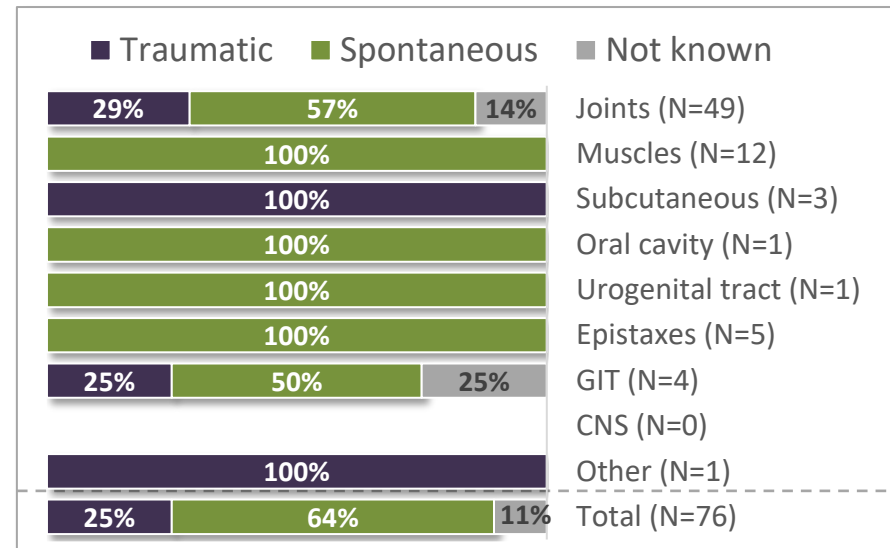
- Joints (N=49)
- Other (N=27)



- Knee (N=11)
- Ankle (N=17)
- Elbow (N=11)
- Other joint (N=10)



- Muscles (N=12)
- Subcutaneous (N=3)
- Oral cavity (N=1)
- Urogenital tract (N=1)
- Epistaxes (N=5)
- GIT (N=4)
- CNS (N=0)
- Other (N=1)



Adults
Haem B
N=76*

Detailed treatment of bleeds

* number of bleeds

	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	49	12	3	1	1	5	4	0	1	76
FIX consumption per bleed (IU), valid N	49	12	3	1	1	5	4		1	76
geometric mean	4598.1	2803.7	28400.5	3200.0	15000.0	3078.5	5305.0		29000.0	4643.9
median	4000.0	3000.0	41000.0	3200.0	15000.0	3000.0	3500.0		29000.0	4000.0
min – max	1000–68000	1000–8000	4800–116400	3200–3200	15000–15000	3000–3200	1200–55000		29000–29000	1000–116400
sum	386300	38800	162200	3200	15000	15400	63200		29000	713100
No. of doses per bleed										
geometric mean	1.9	1.1	11.7	7.0	5.0	1.6	2.8		8.0	2.0
median	1	1	18	7	5	1	2		8	1
min – max	1–17	1–2	2–44	7–7	5–5	1–3	1–15		8–8	1–44
Duration of therapy per bleed, days										
geometric mean	1.8	1.2	7.1	2.0	10.0	1.6	2.3		7.0	1.8
median	1	1	7	2	10	1	2		7	1
min – max	1–16	1–2	2–26	2–2	10–10	1–3	1–14		7–7	1–26
N (%) with hospitalization	1 (2%)	0 (0%)	2 (66.7%)	0 (0%)	0 (0%)	0 (0%)	1 (25%)		0 (0%)	4 (5.3%)
N (%) with rebleeding	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	1 (20%)	0 (0%)		0 (0%)	1 (1.3%)

ABR according to centres Haemophilia B (PWHB)

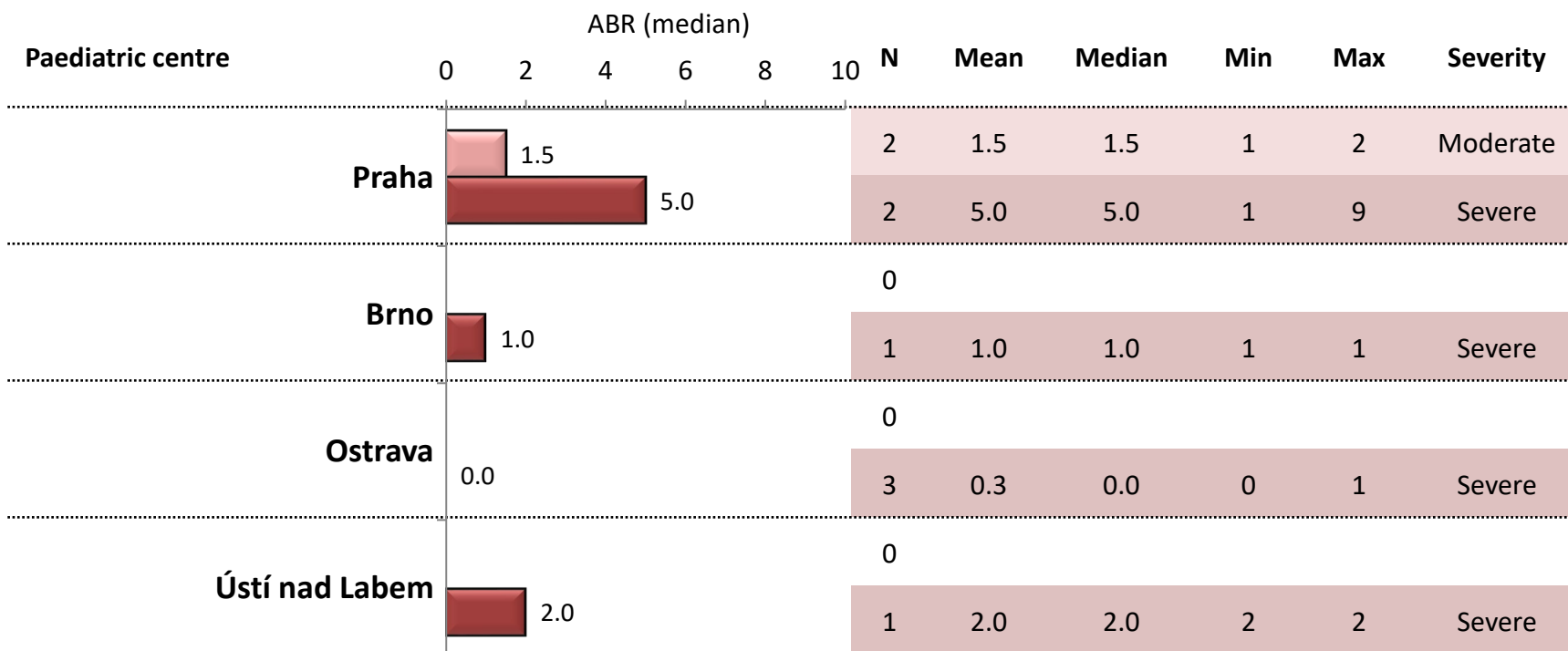


Annual bleeding rate on permanent prophylaxis

HaemB on prophylaxis
Paed. centres
N=9

 Moderate
 Severe

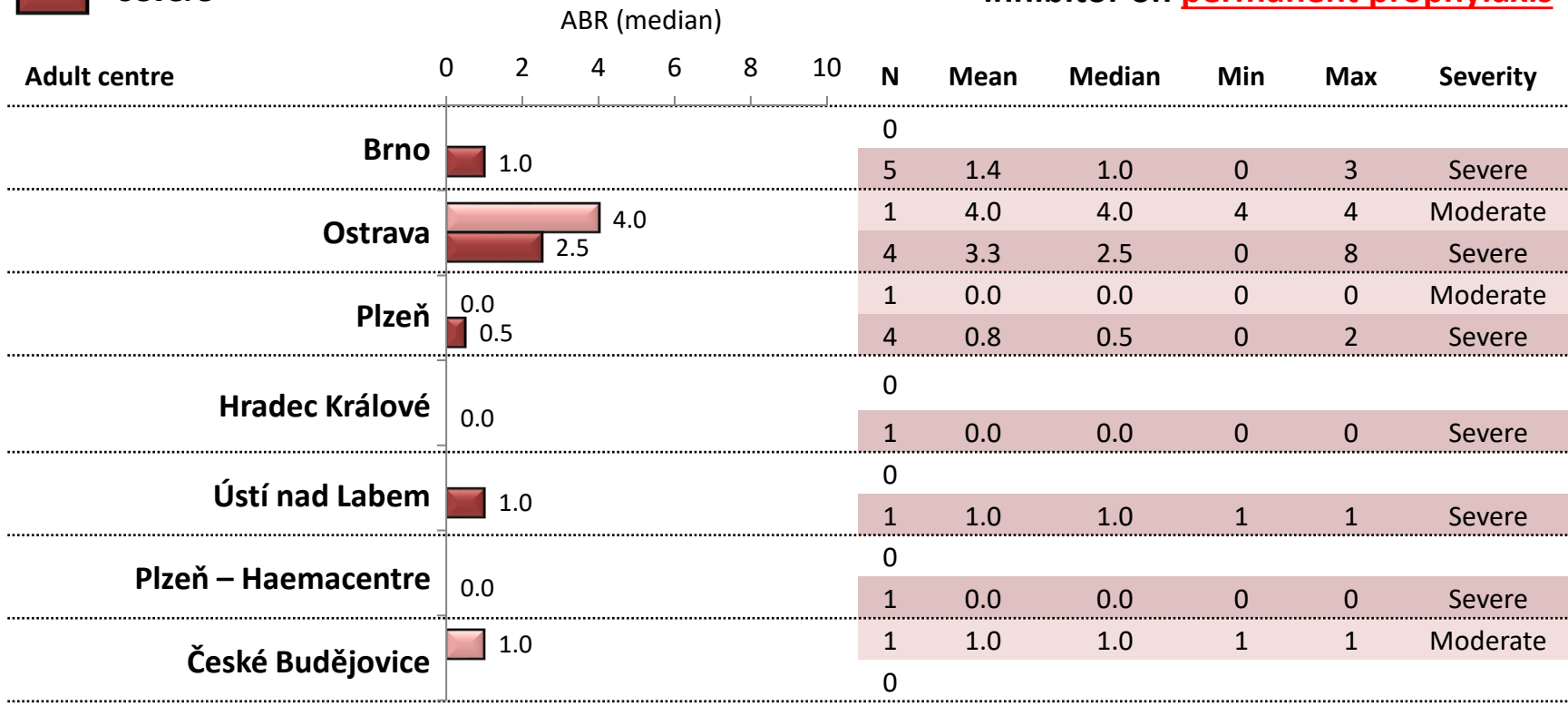
Frequency of bleeding in PWHB without inhibitor on **permanent prophylaxis**



Annual bleeding rate on permanent prophylaxis



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

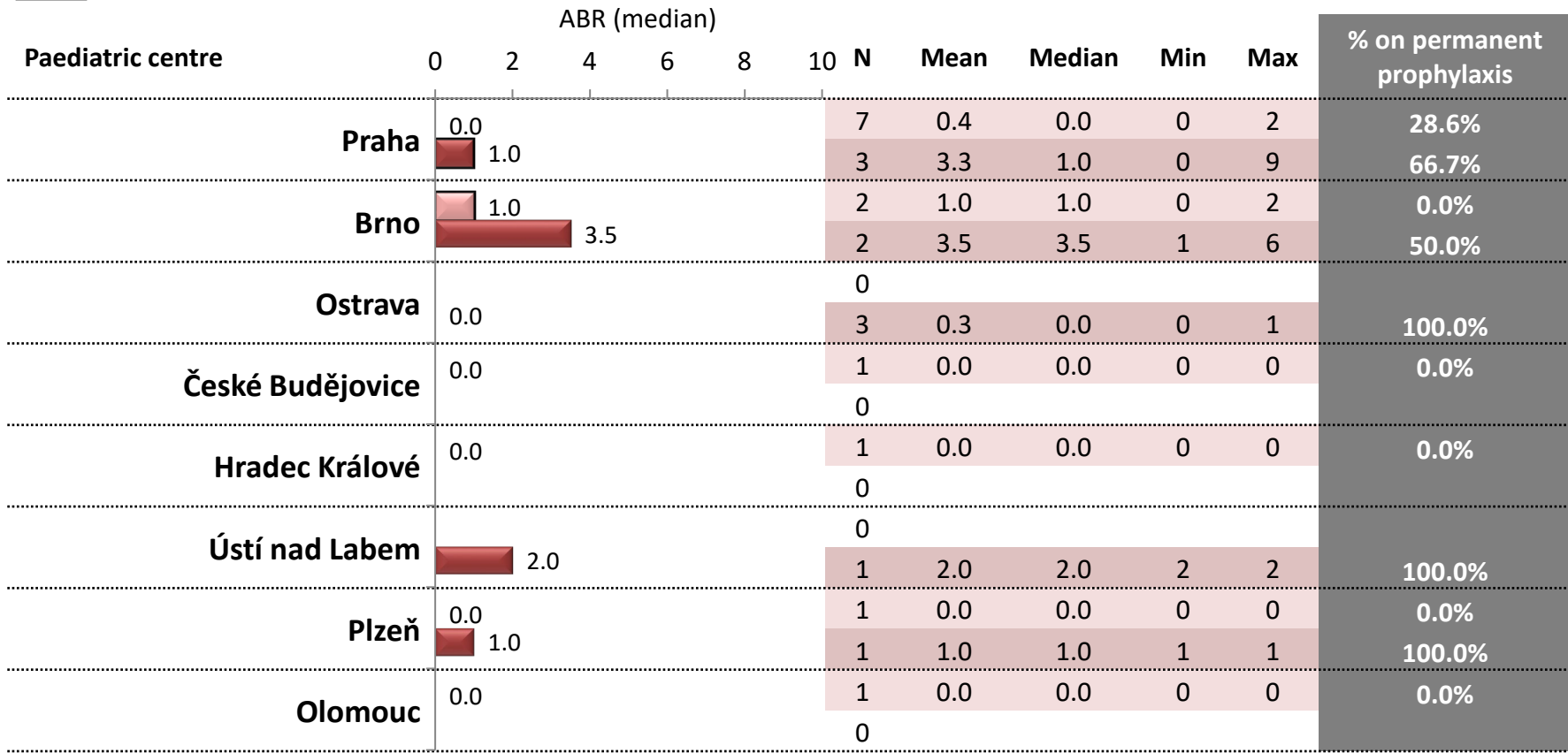


Annual bleeding rate regardless prophylaxis

HaemB
Paed. centres
N=23



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



Annual bleeding rate regardless prophylaxis

HaemB
Adult centres
N=44*

* missing ABR in 2 adults



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

Adult centre	ABR (median)					N*	Mean	Median	Min	Max	% on permanent prophylaxis
	0	5	10	15	20						
Brno	1.0					4	1.3	1.0	0	3	0.0%
	1.0					5	1.4	1.0	0	3	100.0%
Ostrava	2.0					2	2.0	2.0	0	4	50.0%
	0.5					6	2.2	0.5	0	8	66.7%
Plzeň	0.0					3	0.3	0.0	0	1	33.3%
	0.5					4	0.8	0.5	0	2	100.0%
Liberec	0.0					1	0.0	0.0	0	0	0.0%
					16.0	1	16.0	16.0	16	16	0.0%
Olomouc	1.0					8	1.1	1.0	0	4	0.0%
	0.0					1	0.0	0.0	0	0	66.7%
Hradec Králové	0.0					1	0.0	0.0	0	0	0.0%
	2.0					2	2.0	2.0	0	4	50.0%
Ústí nad Labem						0					
	3.0					2	3.0	3.0	1	5	50.0%
Plzeň – Haemacentre						0					
	0.0					1	0.0	0.0	0	0	100.0%
České Budějovice	1.0					1	1.0	1.0	1	1	100.0%
	2.0					2	2.0	2.0	0	4	0.0%

Prophylactic regimens and treatment outcomes

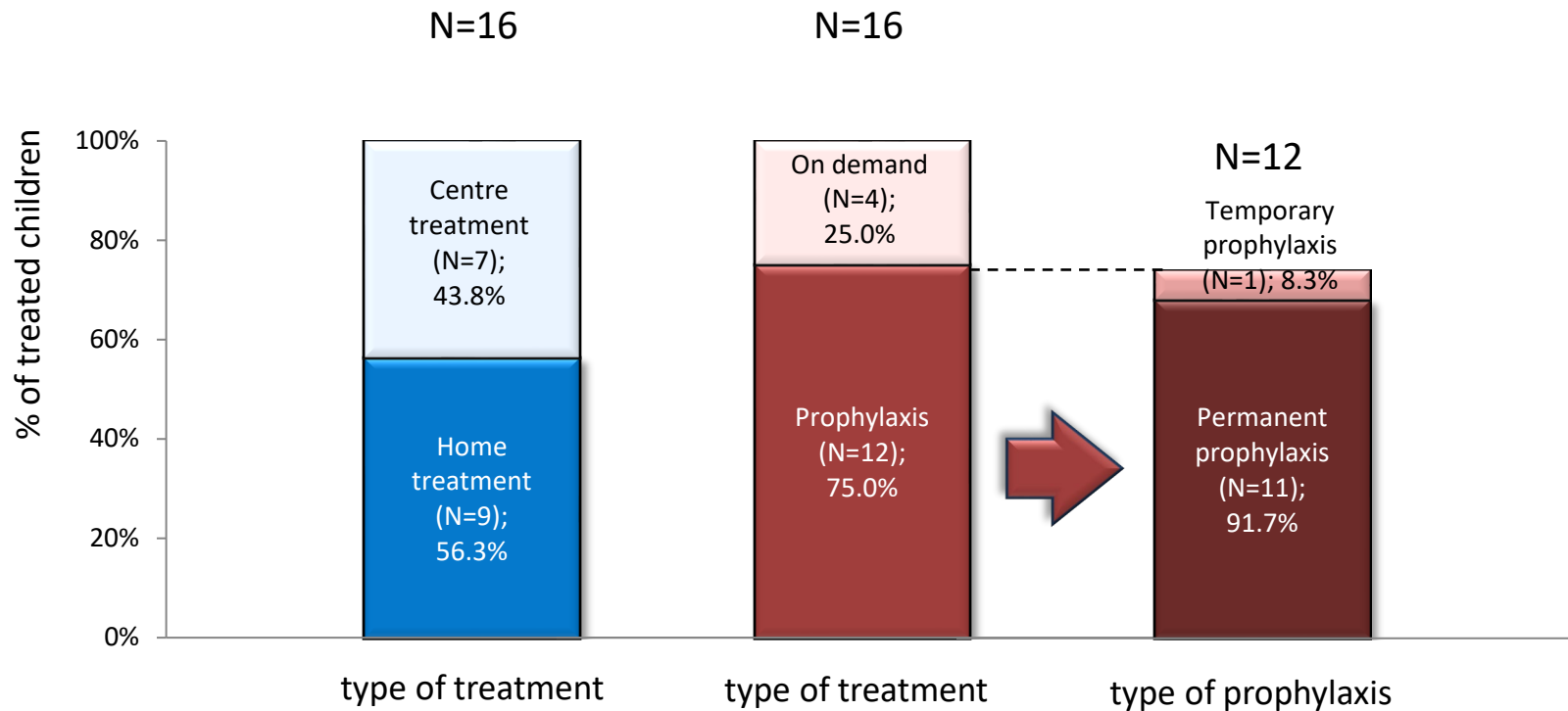
HaemB
Paed. centres
N=23

Paediatric centre	Severity	Total N	PERMANENT PROPHYLAXIS								ON-DEMAND / TEMPORARY PROPHY		
			% of patients	N	Dosing of prophylaxis (IU/kg per week)				ABR		N	ABR	
					Mean	Median	Min	Max	Mean	Median		Mean	Median
Praha	Moderate	7	28.6%	2	42.1	42.1	40.4	43.8	0.0	0.0	5	5.0	15.0
	Severe	3	66.7%	2	39.6	39.6	33.9	45.4	0.0	0.0	1	1.0	38.0
Brno	Moderate	2	0.0%	0							2	2.0	6.0
	Severe	2	50.0%	1	29.7	29.7	29.7	29.7	50.4	50.4	1	1.0	21.0
Ostrava	Moderate	0											
	Severe	3	100.0%	3	63.4	78.0	34.1	78.2	0.0	0.0	0		
Č. Budějovice	Moderate	1	0.0%	0							1	1.0	3.0
	Severe	0											
Hradec Králové	Moderate	1	0.0%	0							1	1.0	4.0
	Severe	0											
Ústí nad Labem	Moderate	0											
	Severe	1	100.0%	1	51.7	51.7	51.7	51.7	0.0	0.0	0		
Plzeň	Moderate	1	0.0%	0							1	1.0	2.0
	Severe	1	100.0%	1	37.0	37.0	37.0	37.0	0.0	0.0	0		
Olomouc	Moderate	1	0.0%	0							1	1.0	3.0
	Severe	0											

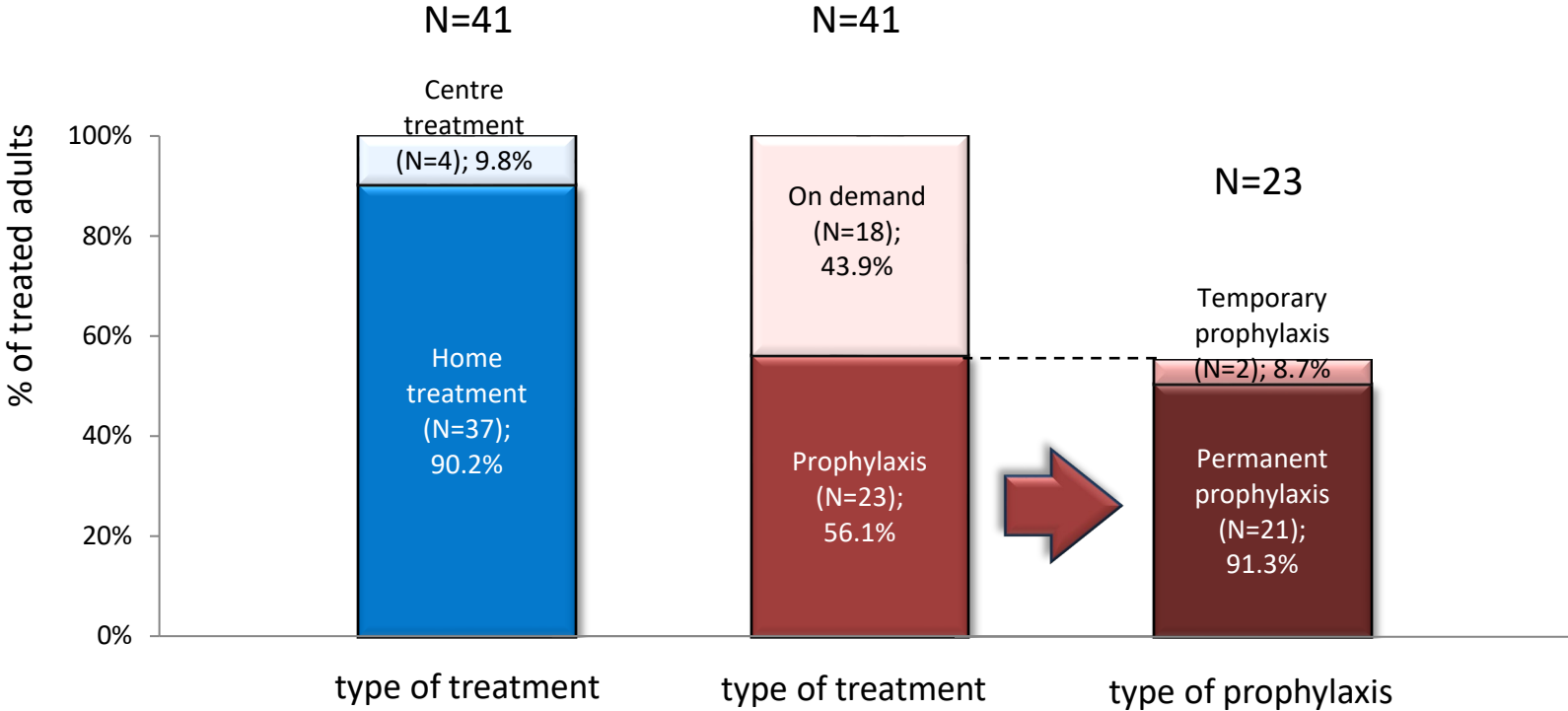
Prophylactic regimens and treatment outcomes

Adult centre	Severity	Total N	PERMANENT PROPHYLAXIS									ON-DEMAND / TEMPORARY PROPHY			
			% of patients	N	Dosing of prophylaxis (IU/kg per week)				ABR*		Age	N	ABR		Age
					Mean	Median	Min	Max	Mean	Median	Median		Mean	Median	Median
Brno	Moderate	4	0.0%	0								4	1.3	1.0	51
	Severe	5	100.0%	5	43.7	45.8	22.9	55.3	1.4	1.0	33	0			
Ostrava	Moderate	2	50.0%	1	50.0	50.0	50.0	50.0	4.0	4.0	21	1	0.0	0.0	37
	Severe	6	66.7%	4	46.7	48.1	25.0	65.6	3.3	2.5	45	2	0.0	0.0	62
Plzeň	Moderate	3	33.3%	1	28.5	28.5	28.5	28.5	0.0	0.0	37	2	0.5	0.5	64
	Severe	4	100.0%	4	29.7	31.1	12.3	44.4	0.8	0.5	41	0			
Liberec	Moderate	1	0.0%	0								1	0.0	0.0	47
	Severe	1	0.0%	0								1	16.0	16.0	28
Olomouc	Moderate	8	0.0%	0								8	1.1	1.0	50
	Severe	3	66.7%	2	31.9	31.9	27.9	35.9	0.0	0.0	53	1	0.0	0.0	28
Hradec Králové	Moderate	1	0.0%	0								1	0.0	0.0	66
	Severe	2	50.0%	1	90.7	90.7	90.7	90.7	0.0	0.0	40	1	4.0	4.0	43
Ústí n. Labem	Moderate	0													
	Severe	2	50.0%	1	52.2	52.2	52.2	52.2	1.0	1.0	25	1	5.0	5.0	49
Plzeň - Haemacentre	Moderate	0													
	Severe	1	100.0%	1	41.7	41.7	41.7	41.7	0.0	0.0	39	0			
Č. Budějovice	Moderate	1	100.0%	1	18.4	18.4	18.4	18.4	1.0	1.0	54	0			
	Severe	2	0.0%	0								2	2.0	2.0	52

Type of treatment (subgroup of treated patients)



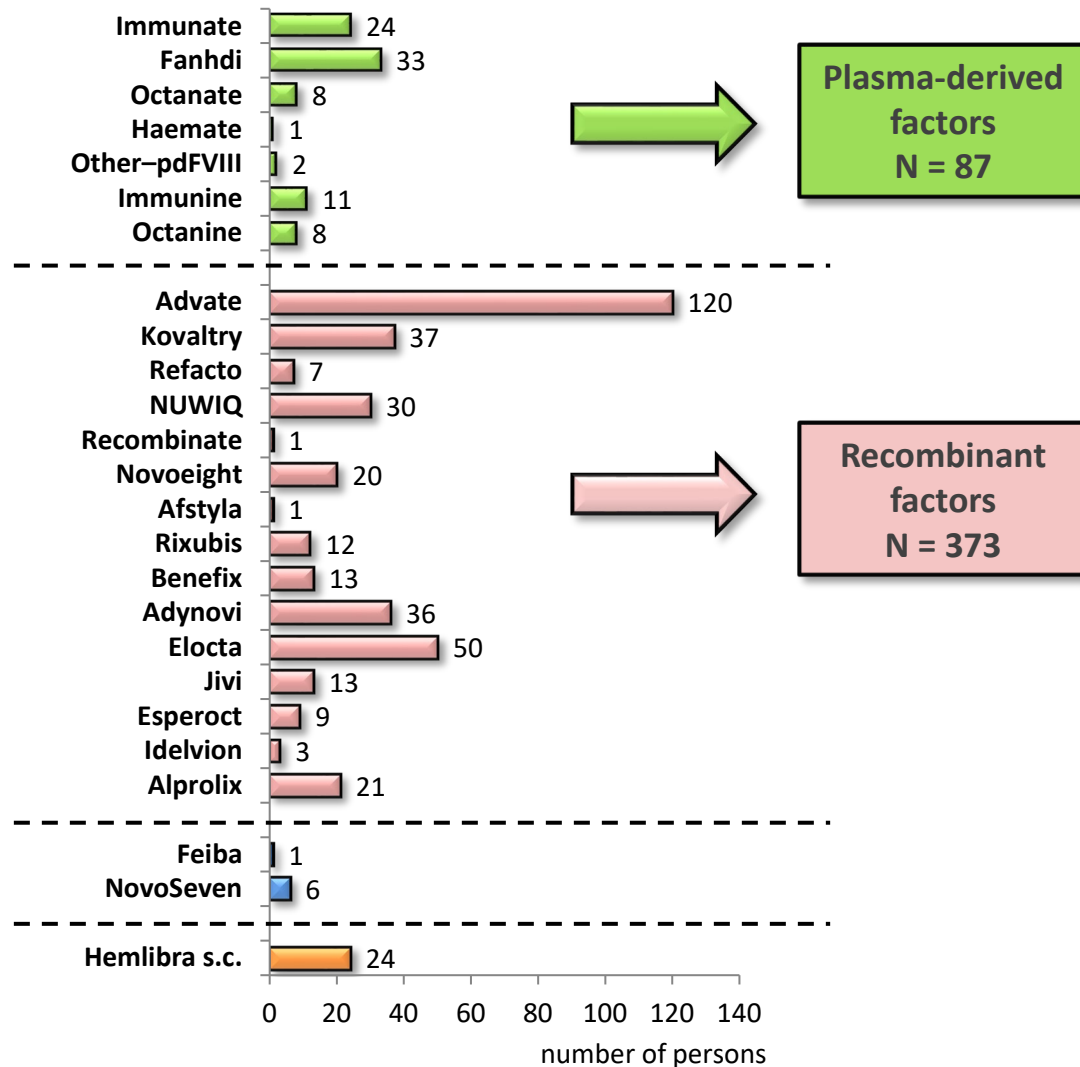
Type of treatment (subgroup of treated patients)



Treatment data and factor consumption

Haemophilia A and B

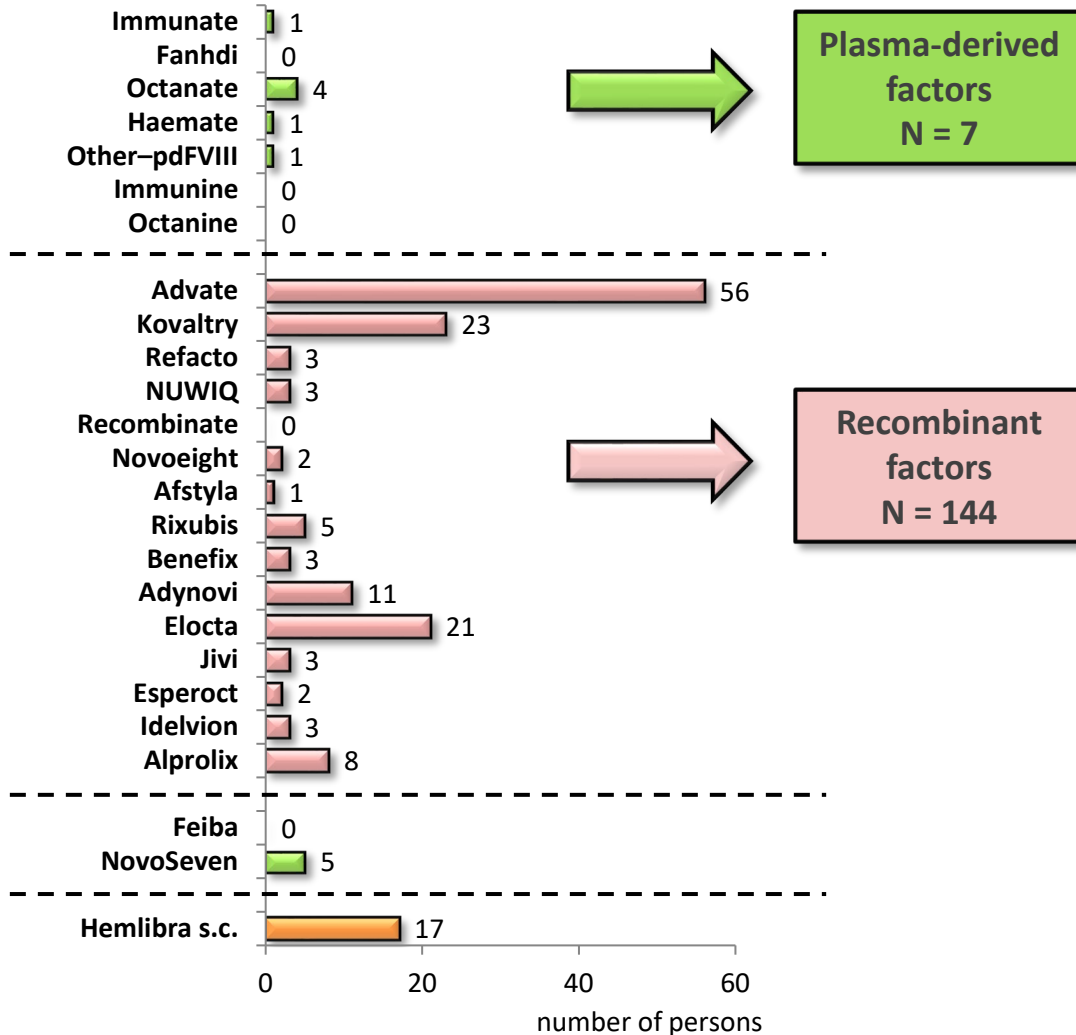
Treatment



404 persons (50.2% of all PWH) were treated in 2020 (**313 persons received standard factor concentrates**, 89 persons received EHL factors, by-pass therapy or emicizumab, in 2 persons data are not available; 72 persons received more than one type/brand of concentrate). 7 persons were treated with both plasma-derived and recombinant factor.

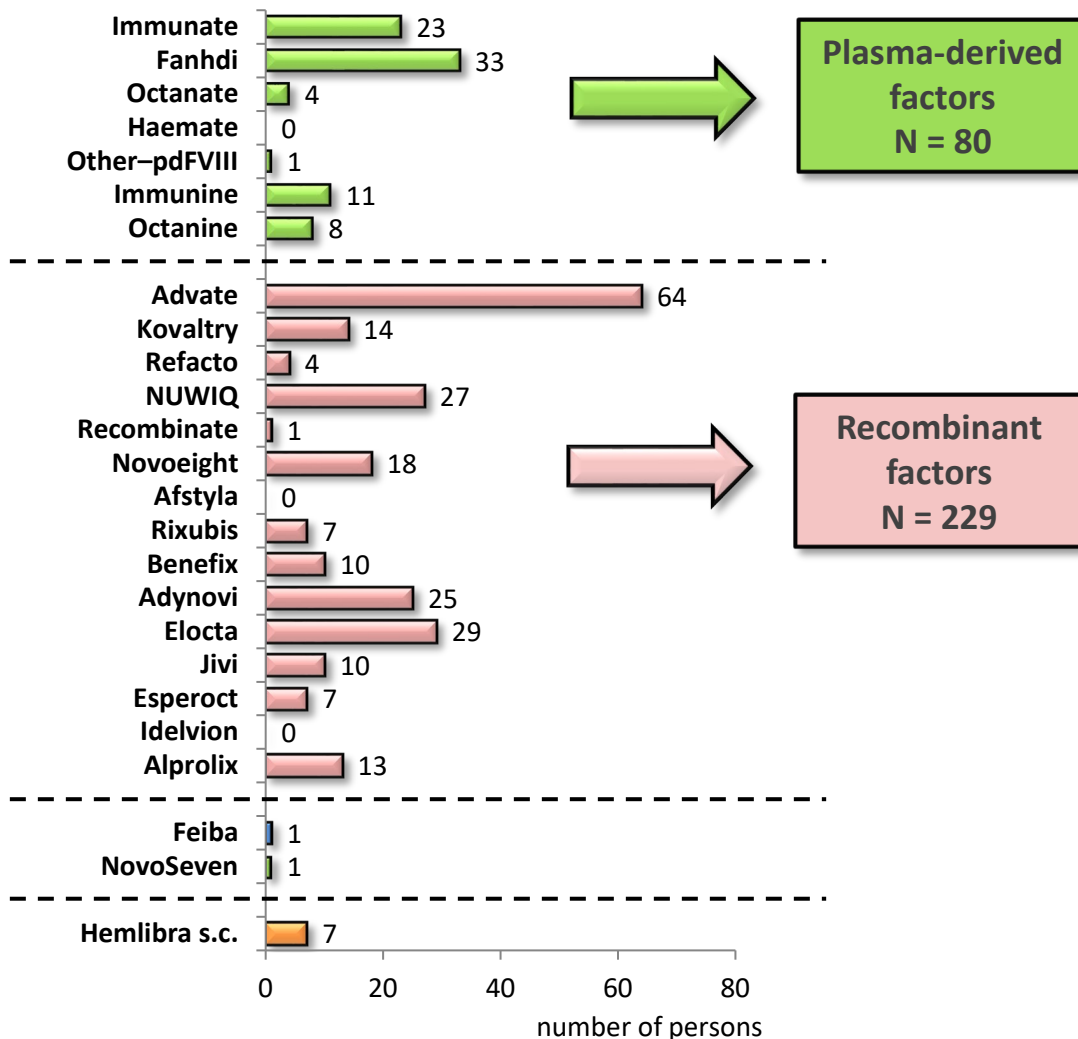
¹missing type of treatment in 2 adults

Treatment



134 children (50.6% of all PWH) were treated in 2020 (**100 children received standard factor concentrates**, 34 children EHL factors, by-pass therapy or emicizumab; 31 children received more than one type/brand of concentrate). Two children were treated with both plasma-derived and recombinant factor.

Treatment



270 adults (50.1% of all PWH) were treated in 2020 (**213 adults received standard factor concentrates**, 55 adults EHL factors, by-pass therapy or emicizumab, in 2 persons data are not available; 41 adults received more than one type/brand of concentrate). 5 adults were treated with both plasma-derived and recombinant factor.

¹missing type of treatment in 2 adults

Comparison of treatment in years 2020 and 2019

	2020			2019		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	383	47.6	100.0	412	51.5	100.0
<i>Plasma-derived factor</i>	86	10.7	22.5	152	19.0	36.9
<i>Recombinant factor</i>	234	29.1	61.1	279	34.9	67.7
<i>Recombinant f. EHL</i>	127	15.8	33.2	68	8.5	16.5
Without treatment	421	52.4	-	388	48.5	-
Total	804	100.0	-	800	100.0	-

* One patient could have more type of factor concentrates.

Comparison of treatment in years 2020 and 2019

	2020			2019		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	122	46.0	100.0	143	54.2	100.0
<i>Plasma-derived factor</i>	7	2.6	5.7	19	7.2	13.3
<i>Recombinant factor</i>	95	35.8	77.9	123	46.6	86.0
<i>Recombinant f. EHL</i>	45	17.0	36.9	26	9.8	18.2
Without treatment	143	54.0	-	121	45.8	-
Total	265	100.0	-	264	100.0	-

* One patient could have more type of factor concentrates.

Comparison of treatment in years 2020 and 2019

	2020			2019		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	261	48.4	100.0	269	50.2	100.0
<i>Plasma-derived factor</i>	79	14.7	30.3	133	24.8	49.4
<i>Recombinant factor</i>	139	25.8	53.3	156	29.1	58.0
<i>Recombinant f. EHL</i>	82	15.2	31.4	42	7.8	15.6
Without treatment	278	51.6	-	267	49.8	-
Total	539	100.0	-	536	100.0	-

* One patient could have more type of factor concentrates.

Consumption of drugs

All

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	<i>Immunate</i>	1 452 500	24	60 520.8
	<i>Fanhdi</i>	2 638 000	33	79 939.4
	<i>Octanate</i>	1 254 450	8	156 806.3
	<i>Haemate P</i>	5 000	1	5 000.0
	<i>Other plasma-derived</i>	51 000	2	25 500.0
	FVIII PD total	5 400 950	67	80 611.2
	<i>Advate</i>	12 158 900	120	101 324.2
	<i>Kovaltry</i>	2 570 500	37	69 473.0
	<i>Refacto</i>	973 000	7	139 000.0
	<i>NUWIQ</i>	3 368 000	28	120 285.7
	<i>Recombinate</i>	2 000	1	2 000.0
	<i>Novoeight</i>	3 292 000	20	164 600.0
	<i>Afstyla</i>	8 000	1	8 000.0
	FVIII REC total	22 372 400	207	108 079.2
	Standard FVIII total	27 773 350	267	104 020.0
	<i>Adynovi</i>	8 259 500	35	235 985.7
	<i>Elocta</i>	6 505 750	50	130 115.0
<i>Jivi</i>	1 633 000	13	125 615.4	
<i>Esperoct</i>	1 351 000	9	150 111.1	
FVIII REC EHL total	17 749 250	104	170 665.9	
FVIII total	45 522 600	325	140 069.5	
FIX (IU)	<i>Immunine</i>	444 000	10	44 400.0
	<i>Octanine</i>	810 500	8	101 312.5
	FIX PD total	1 254 500	18	69 694.4
	<i>Rixubis</i>	1 655 250	12	137 937.5
	<i>Benefix</i>	1 134 500	13	87 269.2
	FIX REC total	2 789 750	25	111 590.0
	Standard FIX total	4 044 250	43	94 052.3
	<i>Idelvion</i>	224 000	3	74 666.7
	<i>Alprolix</i>	1 551 140	21	73 863.8
	FIX REC EHL total	1 775 140	22	80 688.2
FIX total	5 819 390	54	107 766.5	
By-pass	<i>Feiba (U)</i>	556 500	1	556 500.0
	<i>NovoSeven (mg)</i>	1 197.0	6	199.5
Emicizumab	<i>Hemlibra s.c. (mg)</i>	60 224	24	2 509.3

Consumption of drugs

Children

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	<i>Immunate</i>	24 000	1	24 000.0
	<i>Fanhdi</i>	0	0	
	<i>Octanate</i>	605 950	4	151 487.5
	<i>Haemate P</i>	5 000	1	5 000.0
	<i>Other plasma-derived</i>	44 000	1	44 000.0
	FVIII PD total	678 950	7	96 992.9
	<i>Advate</i>	4 573 400	56	81 667.9
	<i>Kovaltry</i>	1 438 500	23	62 543.5
	<i>Refacto</i>	192 000	3	64 000.0
	<i>NUWIQ</i>	992 000	3	330 666.7
	<i>Recombinate</i>	0	0	
	<i>Novoeight</i>	631 500	2	315 750.0
	<i>Afstyla</i>	8 000	1	8 000.0
	FVIII REC total	7 835 400	87	90 062.1
	Standard FVIII total	8 514 350	92	92 547.3
	<i>Adynovi</i>	2 140 000	11	194 545.5
	<i>Elocta</i>	1 784 000	21	84 952.4
	<i>Jivi</i>	371 000	3	123 666.7
	<i>Esperoct</i>	324 000	2	162 000.0
FVIII REC EHL total	4 619 000	36	128 305.6	
FVIII total	11 177 400	104	107 475.0	
FIX (IU)	<i>Immunine</i>	0	0	
	<i>Octanine</i>	0	0	
	FIX PD total	0	0	
	<i>Rixubis</i>	223 250	5	44 650.0
	<i>Benefix</i>	117 500	3	39 166.7
	FIX REC total	340 750	8	42 593.8
	Standard FIX total	340 750	8	42 593.8
	<i>Idelvion</i>	224 000	3	74 666.7
	<i>Alprolix</i>	742 000	8	92 750.0
	FIX REC EHL total	966 000	9	107 333.3
FIX total	1 163 250	15	77 550.0	
By-pass	<i>Feiba (U)</i>	0	0	
	<i>NovoSeven (mg)</i>	1 187.0	5	237.4
<i>Emicizumab</i>	<i>Hemlibra s.c. (mg)</i>	24 026	17	1 413.3

Consumption of drugs

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	<i>Immunate</i>	1 428 500	23	62 108.7
	<i>Fanhdi</i>	2 638 000	33	79 939.4
	<i>Octanate</i>	648 500	4	162 125.0
	<i>Haemate P</i>	0	0	
	<i>Other plasma-derived</i>	7 000	1	7 000.0
	FVIII PD total	4 722 000	60	78 700.0
	<i>Advate</i>	7 585 500	64	118 523.4
	<i>Kovaltry</i>	1 132 000	14	80 857.1
	<i>Refacto</i>	781 000	4	195 250.0
	<i>NUWIQ</i>	2 376 000	25	95 040.0
	<i>Recombinate</i>	2 000	1	2 000.0
	<i>Novoeight</i>	2 660 500	18	147 805.6
	<i>Afstyla</i>	0	0	
	FVIII REC total	14 537 000	120	121 141.7
Standard FVIII total		19 259 000	175	110 051.4
FIX (IU)	<i>Adynovi</i>	6 119 500	24	254 979.2
	<i>Elocta</i>	4 721 750	29	162 819.0
	<i>Jivi</i>	1 262 000	10	126 200.0
	<i>Esperoct</i>	1 027 000	7	146 714.3
	FVIII REC EHL total	13 130 250	68	193 091.9
	FVIII total	32 389 250	218	148 574.5
	<i>Immunine</i>	444 000	10	44 400.0
	<i>Octanine</i>	810 500	8	101 312.5
	FIX PD total	1 254 500	18	69 694.4
	<i>Rixubis</i>	1 432 000	7	204 571.4
<i>Benefix</i>	1 017 000	10	101 700.0	
FIX REC total	2 449 000	17	144 058.8	
Standard FIX total		3 703 500	35	105 814.3
By-pass	<i>Idelvion</i>	0	0	
	<i>Alprolix</i>	809 140	13	62 241.5
	FIX REC EHL total	809 140	13	62 241.5
	FIX total	4 512 640	39	115 708.7
	<i>Feiba (U)</i>	556 500	1	556 500.0
<i>NovoSeven (mg)</i>	10.0	1	10.0	
Emicizumab	<i>Hemlibra s.c. (mg)</i>	36 198	7	5 171.1