The status of care for persons with haemophilia registered within CNHP registry
Annual Report 2019

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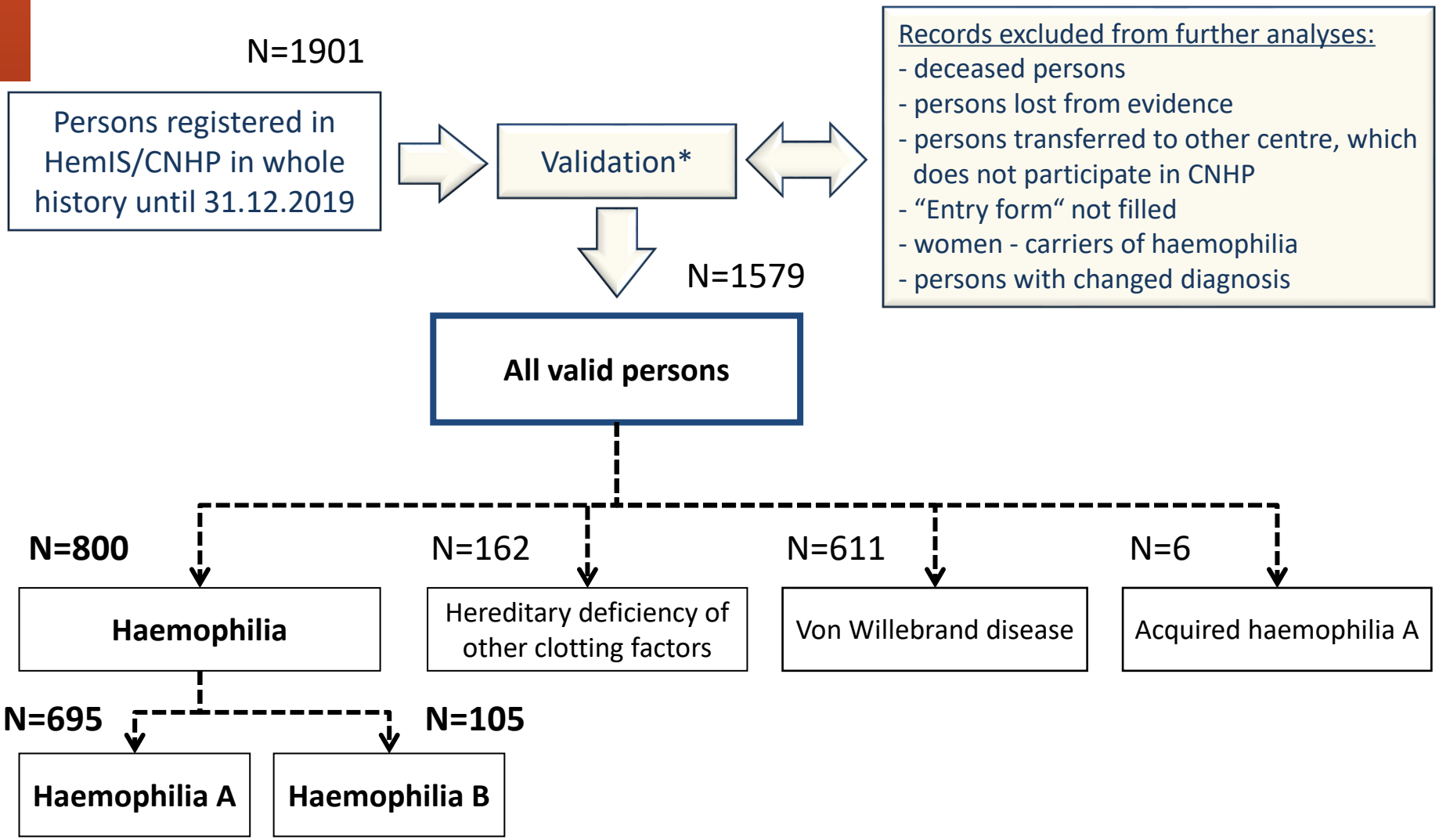
on behalf of

Centres contributing to CNHP registry

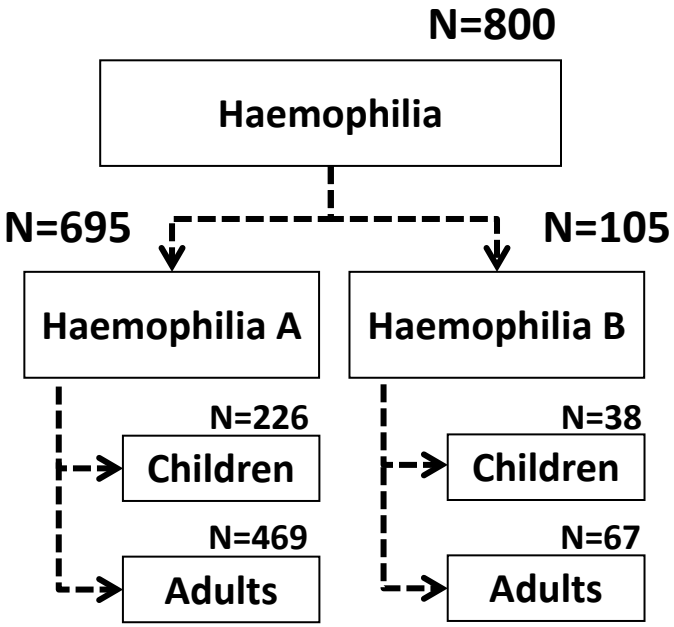
(Czech National Haemophilia Programme)

Export date: May 11, 2020

Sample size, valid records



Persons with haemophilia (PWH)



Centres participating in CNHP

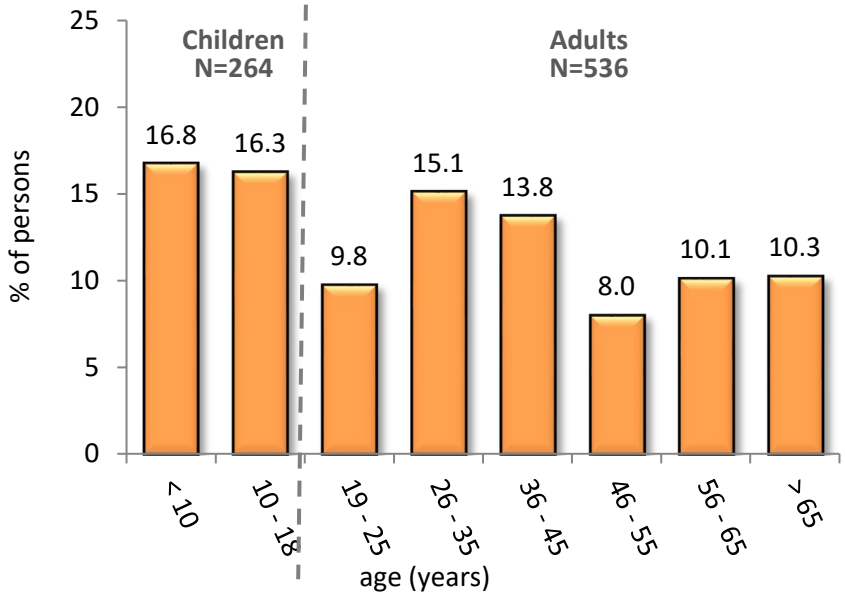
Paediatric centres	Valid persons N	%
Prague – Dpt. of Pediatric Haematology and Oncology, CUH Motol	94	11.8
Brno – Dpt. of Pediatric Haematology, CUH Brno	63	7.9
Hradec Králové – Dpt. of Pediatric Medicine, UH HK	31	3.9
Ostrava – Dpt. of Pediatric Medicine, UH Ostrava	23	2.9
Ústí n.L. – Pediatric Dpt. – Haematology, Masaryk Hospital	22	2.8
Olomouc – Dpt. of Pediatric Medicine, UH Olomouc	18	2.3
České Budejovice – Pediatric Dpt., Hospital CB	17	2.1
Pilsen – Pediatric Dpt., UH Pilsen	15	1.9

Adult centres	Valid persons N	%
Brno – Dpt. Of Clin Hematol, UH Brno	162	20.3
Ostrava – Blood centre, UH Ostrava	75	9.4
Hradec Králové – IV. Internal and Hematology Dpt., UH HK	69	8.6
Olomouc – Haemato-Oncology Dpt., UH Olomouc	59	7.4
Pilsen – Dpt. of Biochemistry and Hematology, UH Pilsen	50	6.3
Liberec – Dpt. Of Clin Hematol, Hospital Liberec	39	4.9
Ústí n.L. – Dpt. Of Clin Hematol, Masaryk Hospital	28	3.5
České Budějovice – Dpt. Of Clin Hematol, Hospital CB	27	3.4
Pilsen - Hemacentrum	8	1.0

Basic demographics

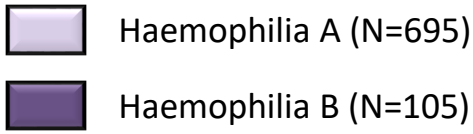
All
N=800

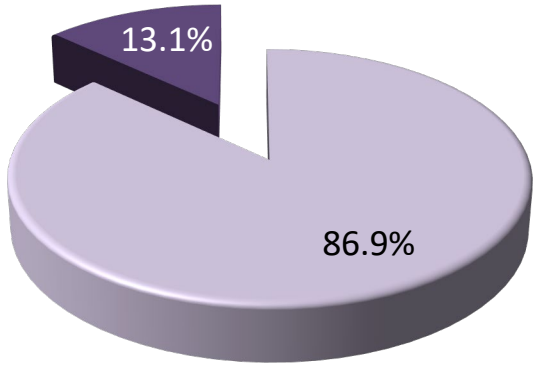
	Actual age* (years)
N	*800*
Mean	*32.9*
Median (min - max)	*30 (0 – 94)*



** age reached in year 2019*

Type of haemophilia





Five children with haemophilia were born in 2019.

All
N=800

Persons with haemophilia and inhibitors in 2019

Active inhibitors were recorded in <u>23 persons</u> in the end of year 2019

- 3 inhibitors in children with severe HA newly developed in 2019 (one of them has developed inhibitor in the end of December 2019 so he is considered as without inhibitor in further analysis)

PWH with inhibitors:

- 14 children and 9 adults
- 22 haemophilia A and 1 haemophilia B
- 19 in severe, 3 in moderate and 1 in mild haemophilia
- 19 high-titre and 3 low-titre (<5BU), 1 value of titre is unknown
- 12 high response and 2 low response inhibitors; this information not available in 8 PWH with inhibitors
- 12 patients were treated with emicizumab
  - 2 patients were treated only with emi, 1 patient with emi and aPCC, 2 patients with emi and rFVIIa, 2 patients with emi and rFVIIa and aPCC during the year
- 3 patients were treated with rFVIIa, 4 patients with aPCC and 2 patients both with rFVIIa and aPCC
  - 4 patients were without any „by-pass“ therapy or emi therapy and 3 patients were without any recorded treatment at all

ITT:

- One child of above mentioned 23 persons started ITT in 2019.
- Another 9 patients have already been on-going ITT in 2019 (started earlier).

Eradication of inhibitor:

- Two children finished ITI successfully during 2019 (they are considered as with inhibitor for 2019).
- Another two children finished ITI unsuccessfully during 2019.

ABR and treatment regimens in patients with inhibitor

N=23

	Type	Year of birth	Severity	ITT	Emi prophylaxis	By-pass prophylaxis	Titre	Responder	ABR	Joint / other
1	HA	2018	Severe	No/NA	Yes	OD	High (>5 BU/ml)	HR	1	1 / 0
2	HA	2018	Severe	Yes	Yes *	OD	NA	NA	0	0 / 0
3	HA	2017	Severe	No/NA		OD	High (>5 BU/ml)	LR	0	0 / 0
4	HA	2017	Severe	Yes		OD	Low	NA	0	0 / 0
5	HA	2017	Severe	Yes		OD	Low	NA	3	1 / 2
6	HA	2016	Severe	Yes		OD	High (>5 BU/ml)	HR	1	0 / 1
7	HA	2016	Severe	Yes	Yes *	OD	High (>5 BU/ml)	LR	2	2 / 0
8	HA	2015	Severe	Yes	Yes *	OD	High (>5 BU/ml)	LR	0	0 / 0
9	HA	2015	Severe	Yes	Yes	OD	High (>5 BU/ml)	HR	4	3 / 1
10	HA	2015	Severe	Yes		OD	High (>5 BU/ml)	NA	0	0 / 0
11	HA	2011	Mild	Yes	Yes *	OD	High (>5 BU/ml)	HR	10	1 / 9
12	HA	2004	Severe	No/NA	Yes	OD	High (>5 BU/ml)	HR	0	0 / 0
13	HA	2003	Severe	No/NA	Yes	OD	High (>5 BU/ml)	HR	4	4 / 0
14	HA	2001	Severe	No/NA	Yes	OD	High (>5 BU/ml)	HR	1	1 / 0
15	HA	1988	Mild	No/NA		OD	Low	NA	0	0 / 0
16	HA	1977	Severe	No/NA		OD	High (>5 BU/ml)	NA	0	0 / 0
17	HA	1975	Severe	No/NA	Yes	OD	High (>5 BU/ml)	HR	2	2 / 0
18	HA	1971	Severe	No/NA	Yes	OD	High (>5 BU/ml)	HR	3	0 / 3
19	HA	1971	Severe	No/NA		OD	High (>5 BU/ml)	NA	0	0 / 0
20	HA	1956	Severe	No/NA		Permanent	High (>5 BU/ml)	HR	0	0 / 0
21	HA	1949	Moderate	No/NA		OD	High (>5 BU/ml)	NA	0	0 / 0
22	HA	1941	Moderate	Yes		Temporary	High (>5 BU/ml)	HR	1	0 / 1
23	HB	2007	Severe	No/NA		Permanent	High (>5 BU/ml)	HR	20	11 / 9

* Emi prophylaxis started after ITT.

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 2019

NA – not available

ABR according to treatment regimen in PWH with inhibitor

N=23

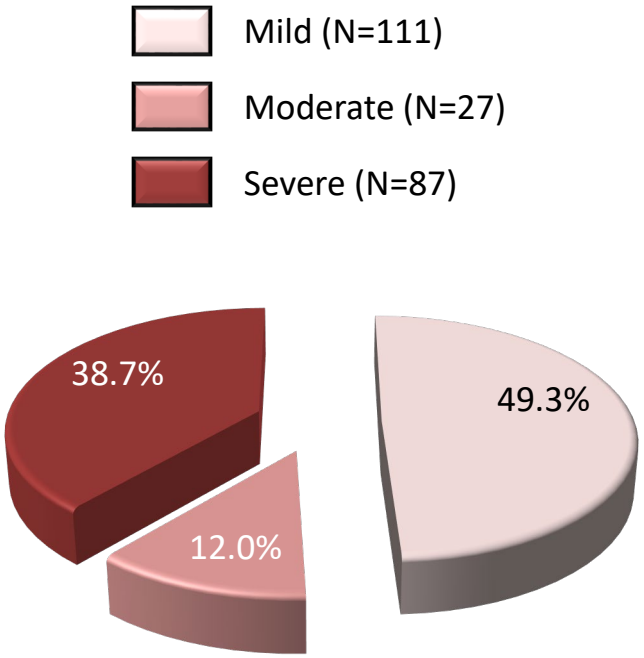
Diagnosis	ITT	Emi/by-pass prophylaxis	N	ABR (mean)	ABR (median, min-max)	Joint / other bleeds (median)
Haemophilia A	Yes	Emi permanent	5	3.20	2 (0-10)	1 / 0
		BPA temporary	1	1.00	1 (1-1)	0 / 1
		OD	4	1.00	0.5 (0-3)	0 / 0.5
	No	Emi permanent	6	1.83	1.5 (0-4)	1 / 0
		BPA permanent	1	0.00	0 (0-0)	0 / 0
		OD	5	0.00	0 (0-0)	0 / 0
Haemophilia B	No	BPA permanent	1	20.00	20 (20-20)	11 / 9

Demographic characteristics Haemophilia A

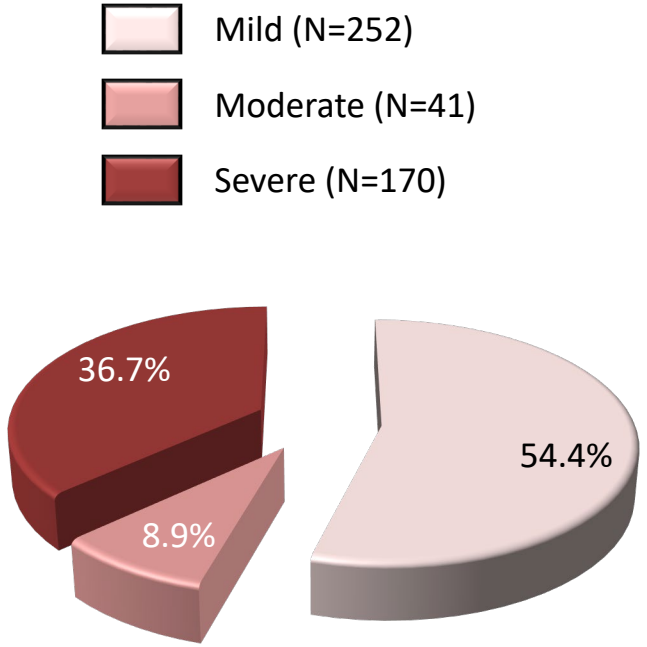
Severity of haemophilia A

Haem A
N=688*

Children (N=225*)



Adults (N=463*)

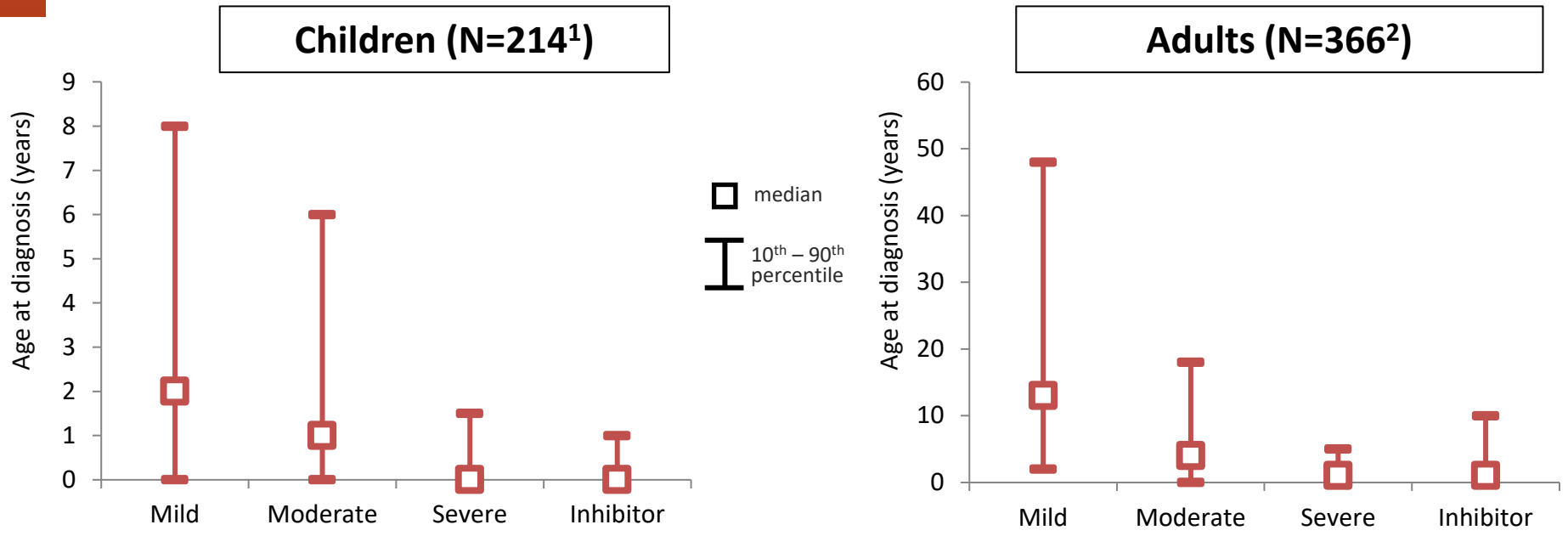


** severity of haemophilia not known in 1 child and 6 adults with haemophilia A*

Age at diagnosis according to severity of haemophilia A

Haem A
N=688*

** severity of haemophilia not known in 1 child and 6 adults*



Mild*	Moderate*	Severe*	Inhibitor+	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor+
108	*26*	*80*	*13*	**N valid**	*209*	*32*	*125*	*8*
2.7	*2.0*	*0.6*	*0.5*	**Mean**	*19.6*	*7.3*	*2.2*	*3.3*
2 (0 – 10)	*1 (0 – 11)*	*0 (0 – 7)*	*0 (0 – 4)*	**Median (min – max)**	*13 (0 – 68)*	*4 (0 – 32)*	*1 (0 – 38)*	*1.5 (0 – 10)*

[1] Missing information on year of diagnosis in 11 children.

[2] Missing information on year of diagnosis in 97 adults.

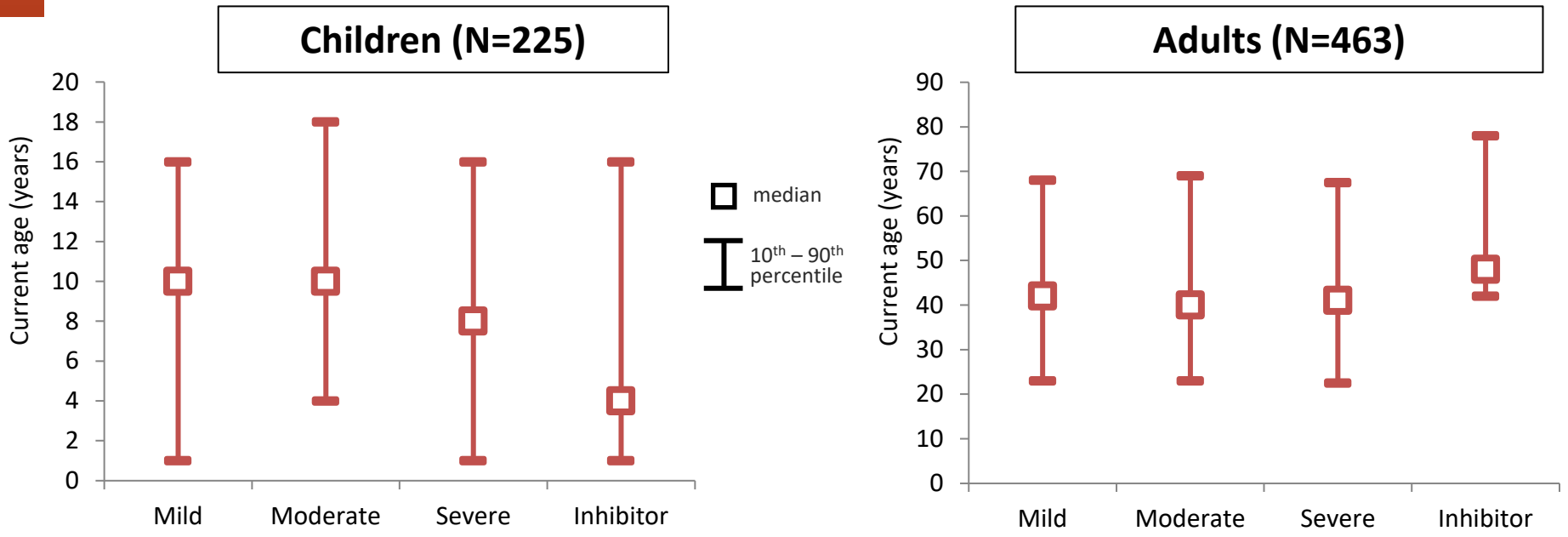
** including persons with inhibitor*

+ in 2019

Actual age according to severity of haemophilia A

Haem A
N=688*

** severity of haemophilia not known in 1 child and 6 adults*



Mild*	Moderate*	Severe*	Inhibitor+	Current age++ (years)	Mild*	Moderate*	Severe*	Inhibitor+
111	*27*	*87*	*13*	**N valid**	*252*	*41*	*170*	*8*
9.5	*10.2*	*8.3*	*6.2*	**Mean**	*45.0*	*43.0*	*43.1*	*53.0*
10 (0 – 18)	*10 (1 – 18)*	*8 (0 – 18)*	*4 (0 – 18)*	**Median (min – max)**	*42 (19 – 89)*	*40 (20 – 78)*	*41 (19 – 80)*	*48 (31 – 78)*

** including persons with inhibitor*

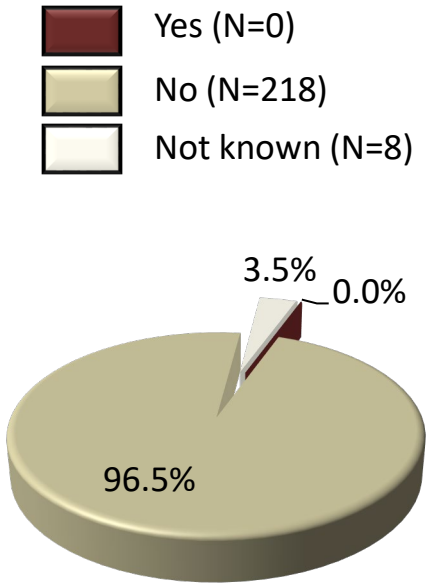
+ in 2019

++ age reached in year 2019

Hepatitis (ever) experienced

Children
Haem A
N=226

Experienced hepatitis



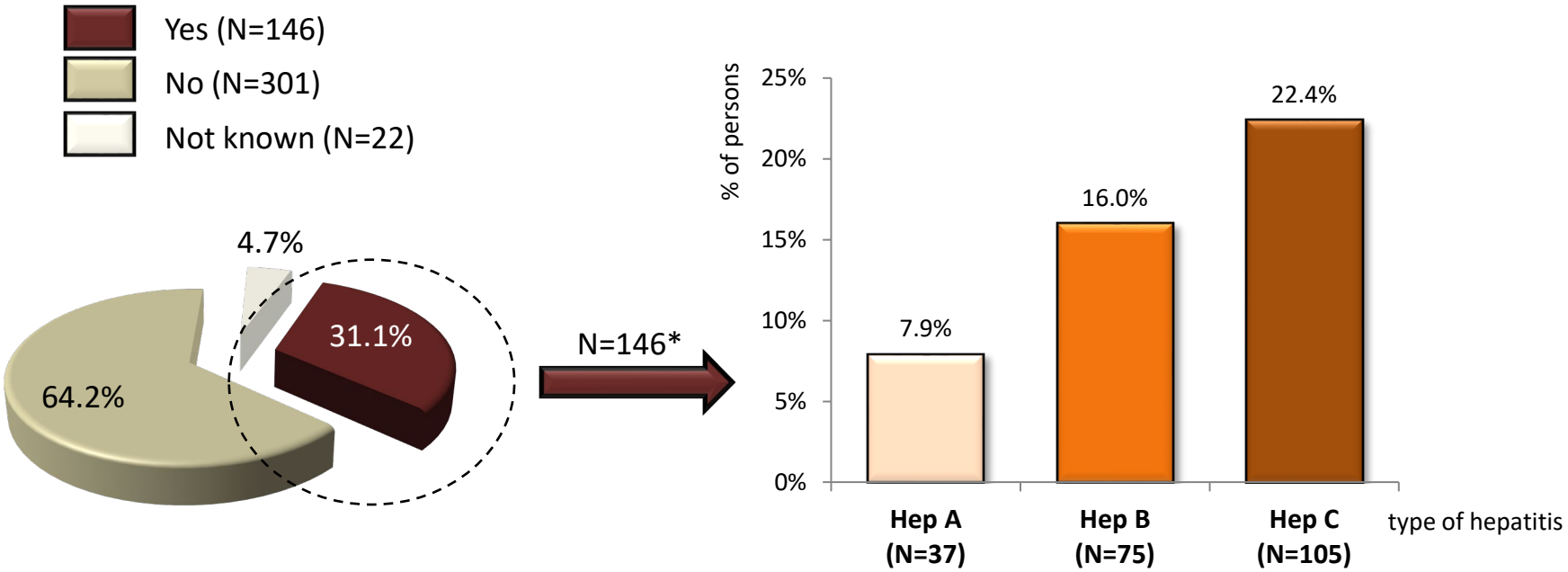
No child has hepatitis.

Data from last completed annual report of each person.

Hepatitis (ever) experienced

Adults
Haem A
N=469

Experienced hepatitis



Data from last completed annual report of each person.

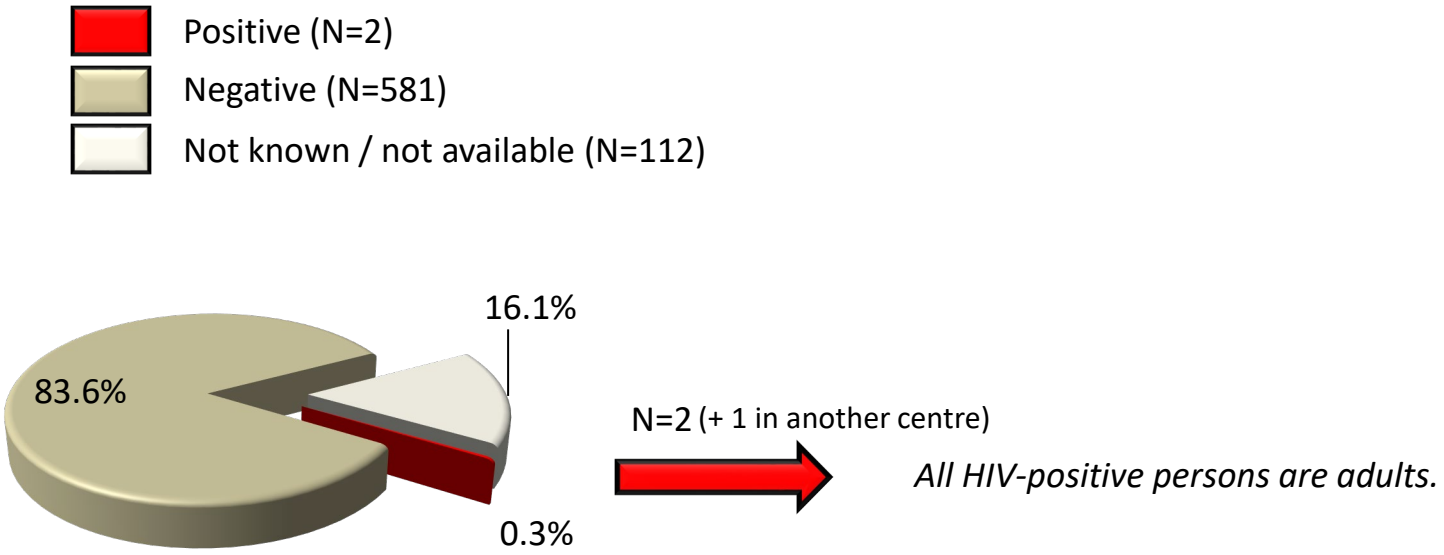
**Total of 217 cases of hepatitis in 146 persons. One person may have more types of hepatitis recorded.*

25 adults are HCV RNA positive

HIV

All
Haem A
N=695

HIV



Data from last completed annual report of each person.

Treatment outcomes and bleeding frequency Haemophilia A

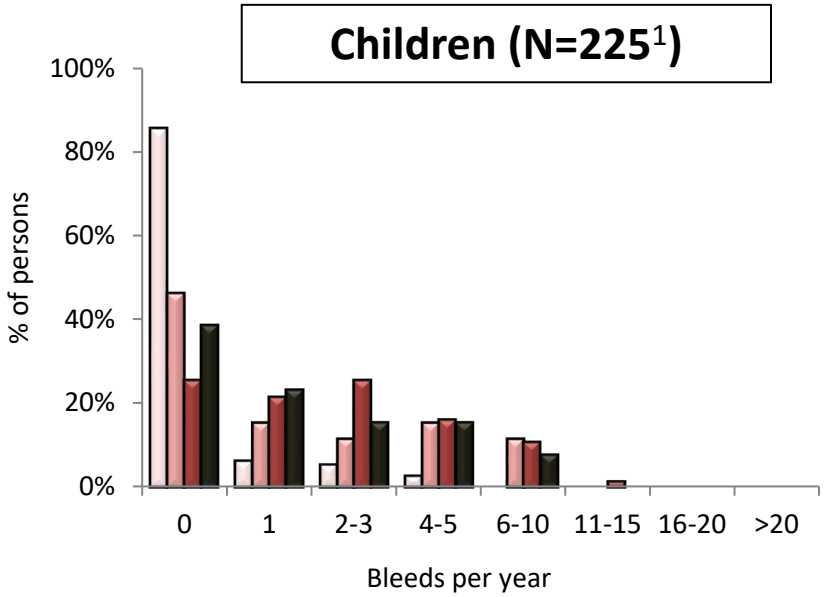
All
Haem A
N=695

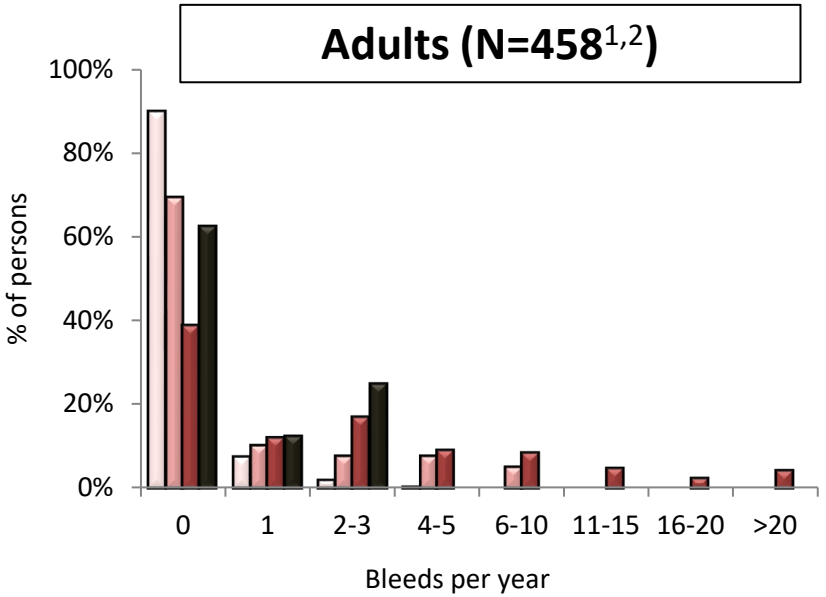
Data from year 2019 – sample size

	Valid persons			Persons with valid annual report			Persons examined			Persons treated	
	N	%		N	%		N	%		N	%
All	695	100%	→	639	91.9%	→	484	69.6%	→	360	51.8%
of them with inhibitor	21			19			18			18	
Children	226	100%	→	208	92.0%	→	193	85.4%	→	129	57.1%
of them with inhibitor	13			12			13			13	
Adults	469	100%	→	431	91.9%	→	291	62.0%	→	231	49.3%
of them with inhibitor	8			7			5			5	

Haem A
N=683[1]

Frequency of bleeding requiring treatment in 2019





Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
111	*26*	*75*	*13*	**N valid**	*251*	*39*	*160*	*8*
0.3	*1.9*	*2.5*	*2.0*	**Mean**	*0.1*	*1.1*	*4.2*	*0.8*
0 (0 – 4)	*1 (0 – 7)*	*2 (0 – 11)*	*1 (0 – 10)*	**Median (min – max)**	*0 (0 – 4)*	*0 (0 – 9)*	*1 (0 – 51)*	*0 (0 – 3)*
95 (85.6%)	*12 (46.2%)*	*19 (25.3%)*	*5 (38.5%)*	**N (%) with no bleed**	*226 (90%)*	*27 (69.2%)*	*64 (38.8%)*	*5 (62.5%)*

** without inhibitor*

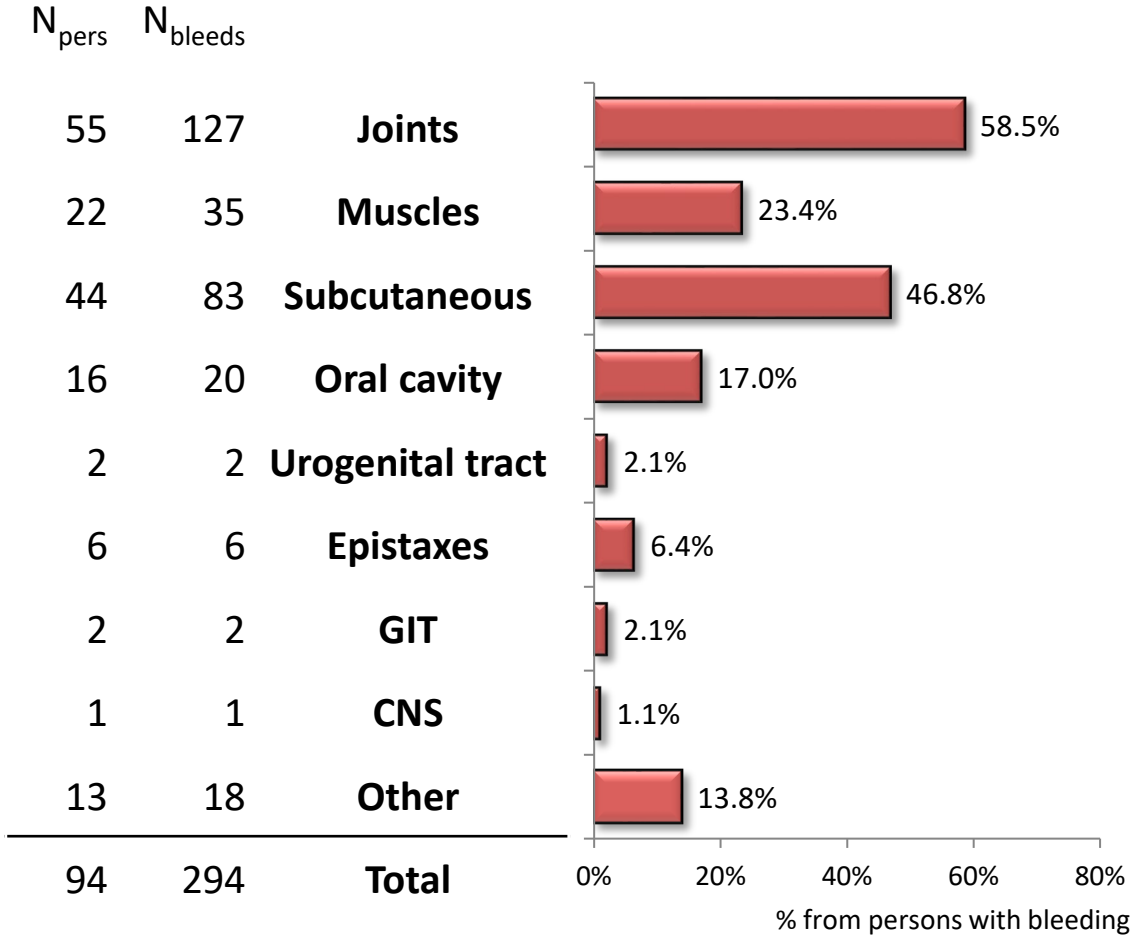
[1] Missing severity in 1 child and 6 adults.
[2] Frequency of bleeding is missing in 5 adults.

Location of bleeds in 2019

Children
Haem A
N=226

94 (41.6%) children experienced bleeding requiring treatment at least once in year; 294 bleeds were recorded in total, 22 bleeds required hospitalization.
All of these 94 children have recorded location of their bleeds.

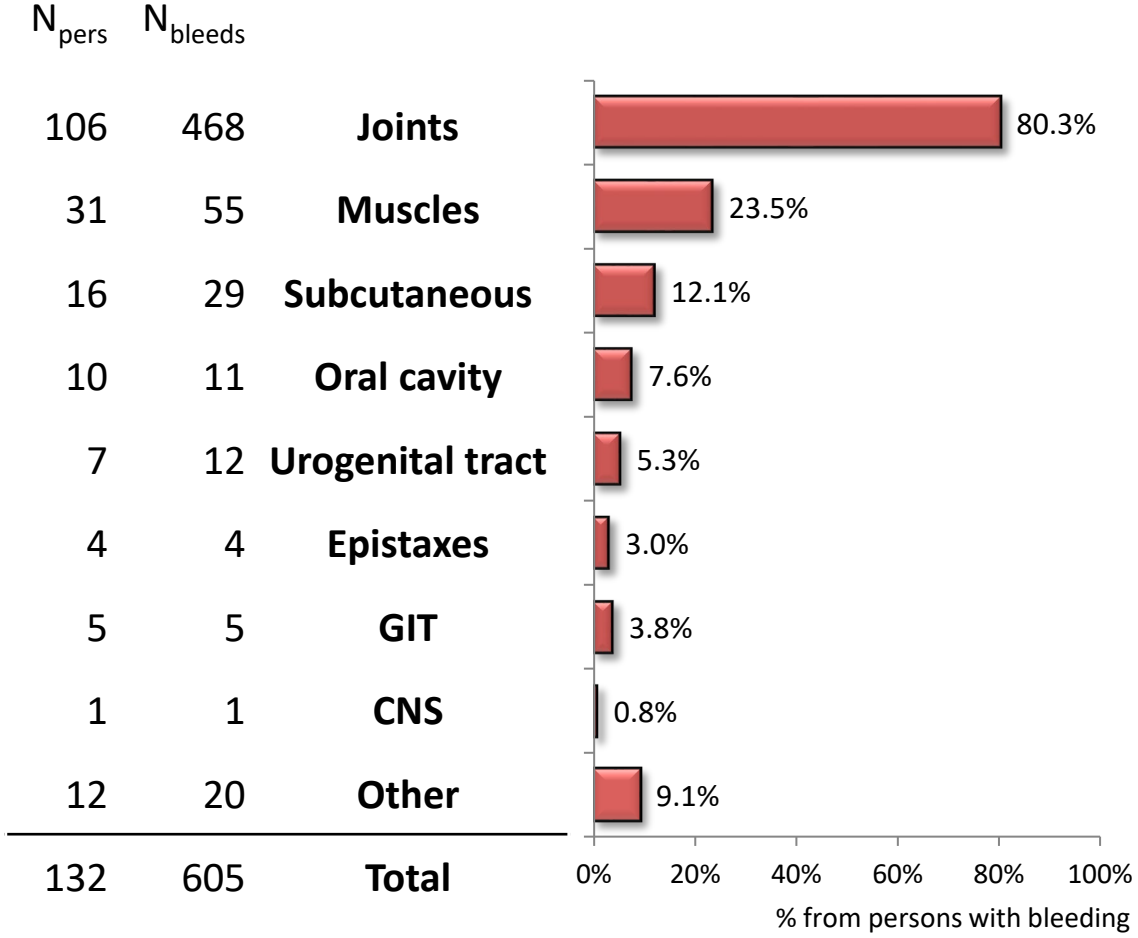
132 (58.4%) children recorded no bleed during year 2019.



Location of bleeds in 2019

Adults
Haem A
N=464[1]

136 (29.3%) adults experienced bleeding requiring treatment at least once in year; 746 bleeds were recorded in total, 20 bleeds required hospitalization.
132 of these 136 adults have recorded location of their bleeds. Localization is not known in 4 adults.
328 (70.7%) adults have recorded no bleed during year 2019.

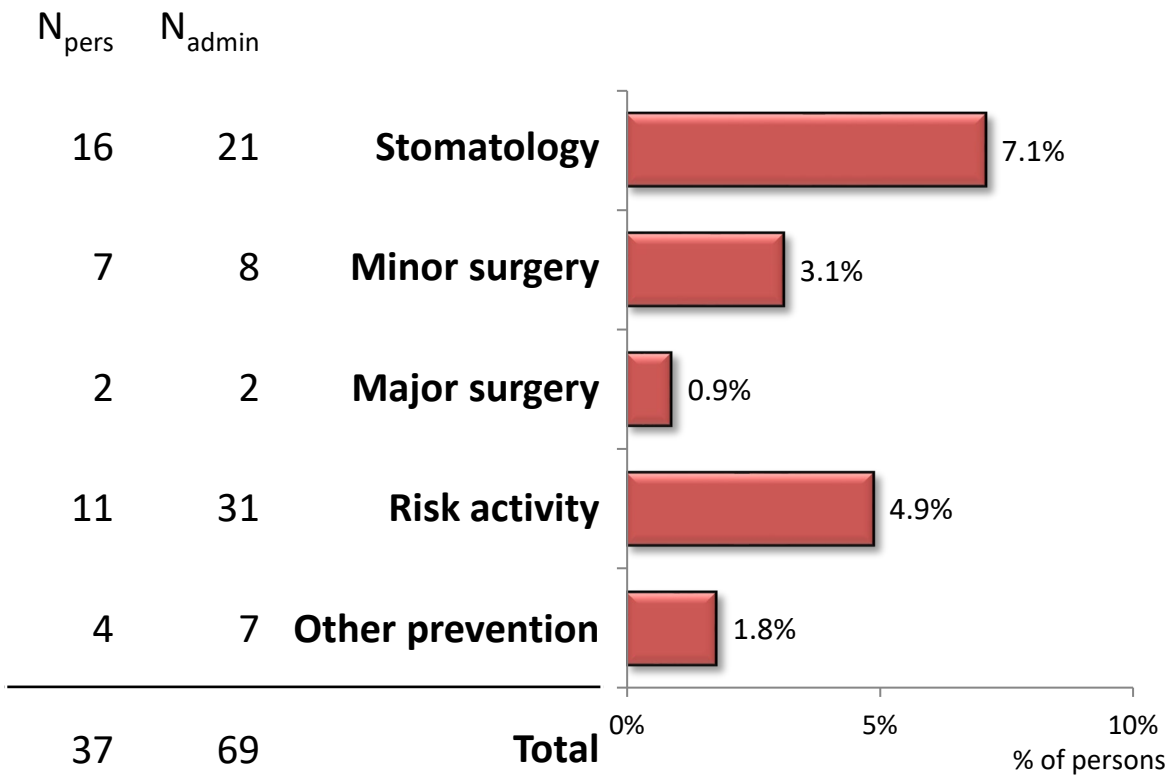


[1]*Frequency of bleeding is missing in 5 adults.*

Preventive administration in 2019

Children
Haem A
N=226

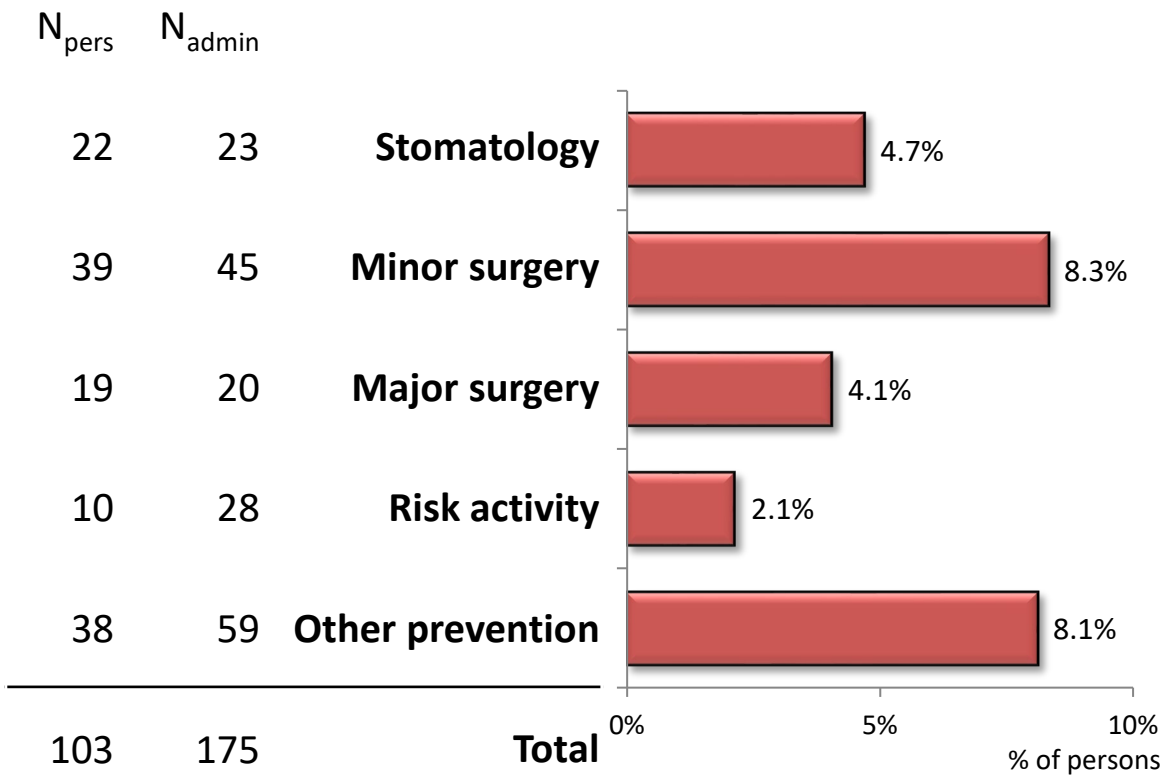
37 (16.4%) children were given factor to prevent bleeding during/before risk situation.
69 preventive administrations were recorded in total.



Preventive administration in 2019

Adults
Haem A
N=469

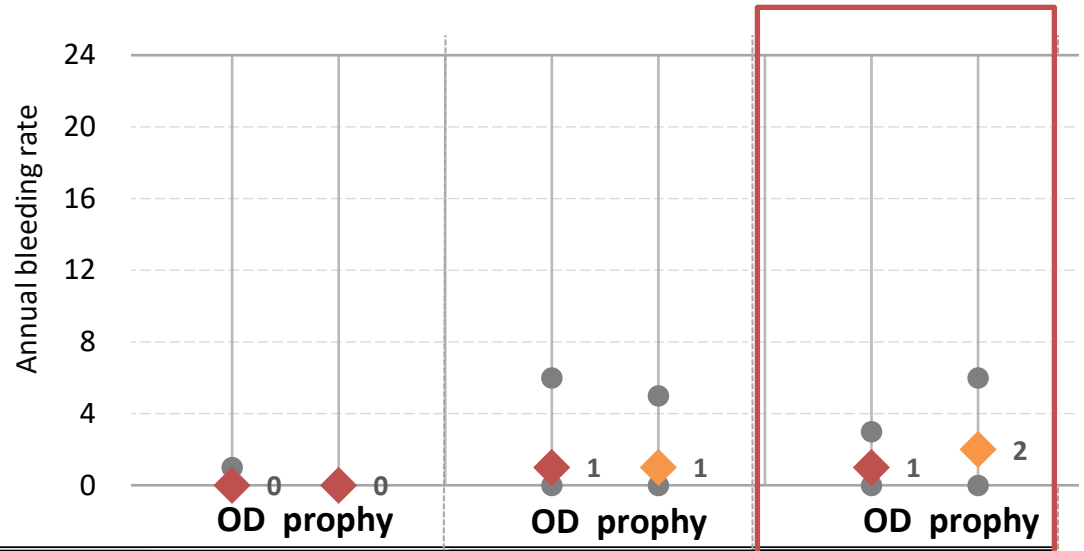
103 (22.0%) persons were given factor to prevent bleeding during/before risk situation.
175 preventive administrations were recorded in total.



ABR according to treatment regimen Haemophilia A without inhibitor

Annual bleeding rate according to treatment regimen

Children Haem A N=212*



Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	111	0	18	8	10	65
Mean	0.3		1.9	1.9	**1.1**	**2.7**
Median (min – max)	0 (0 – 4)		1 (0 – 7)	1 (0 – 5)	**1 (0 – 4)**	**2 (0 – 11)**
Total no of recorded bleeds	33		34	15	**11**	**175**
children on permanent prophylaxis	**0 (0%)**		**8 (30.8%)**		**65 (86.7%)**	
% of factor (FVIII) consumed by children on permanent prophylaxis	-		**91.3%**		**98.8%**	

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

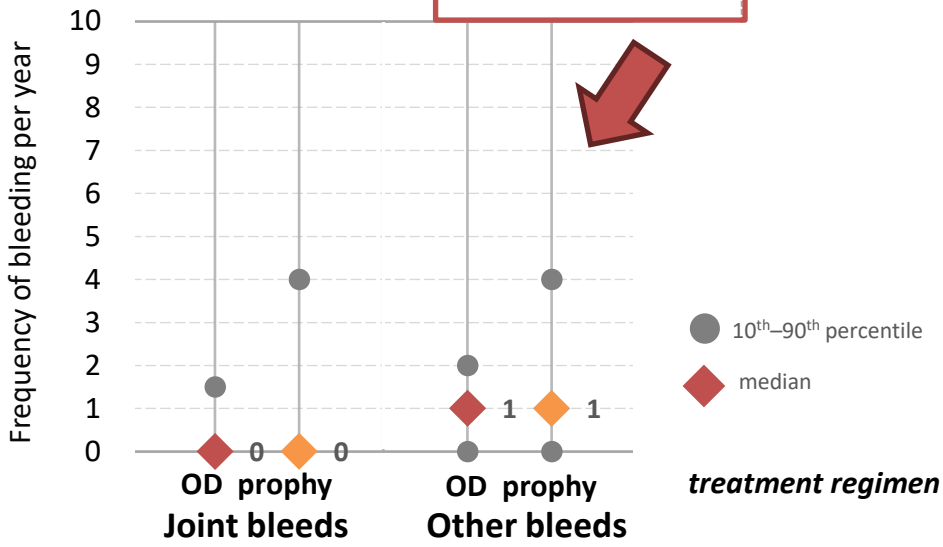
** without inhibitor; missing severity in 1 child*

Joint and other bleeds according to treatment regimen

Children Haem A N=212*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	111	0	18	8	10	65
JOINT BLEEDS						
Mean	0.1		0.6	0.9	**0.3**	**1.2**
Median (range)	0 (0 – 3)		0 (0 – 3)	0 (0 – 5)	**0 (0 – 2)**	**0 (0 – 9)**
Total no of recorded bleeds	11		11	7	**3**	**81**
OTHER BLEEDS						
Mean	0.2		1.3	1.0	**0.8**	**1.4**
Median (range)	0 (0 – 3)		0 (0 – 6)	0 (0 – 5)	**1 (0 – 2)**	**1 (0 – 9)**
Total no of recorded bleeds	21		23	8	**8**	**94**

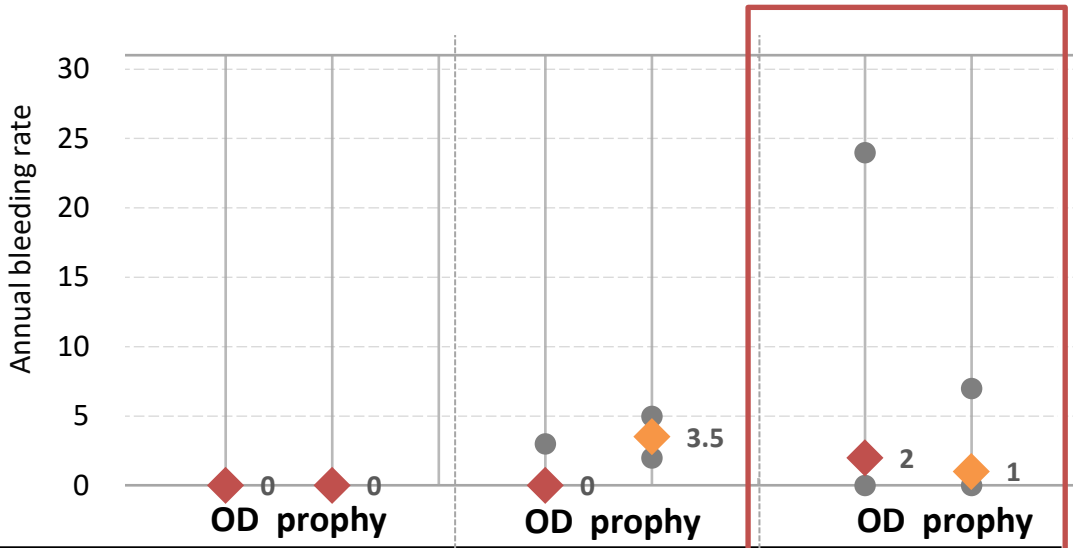
** without inhibitor; missing severity in 1 child*



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

Annual bleeding rate according to treatment regimen

Adults
Haem A
N=450*



Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	251	0	35	4	47	113
Mean	0.1		0.8	3.5	**7.0**	**3.0**
Median (min – max)	0 (0 – 4)		0 (0 – 9)	3.5 (2 – 5)	**2 (0 – 48)**	**1 (0 – 51)**
Total no of recorded bleeds	33		28	14	**328**	**337**
adults on permanent prophylaxis	**0 (0%)**		**4 (10.3%)**		**113 (68.5%)**	
% of factor (FVIII) consumed by adults on permanent prophylaxis	-		**76.3%**		**91.2%**	

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

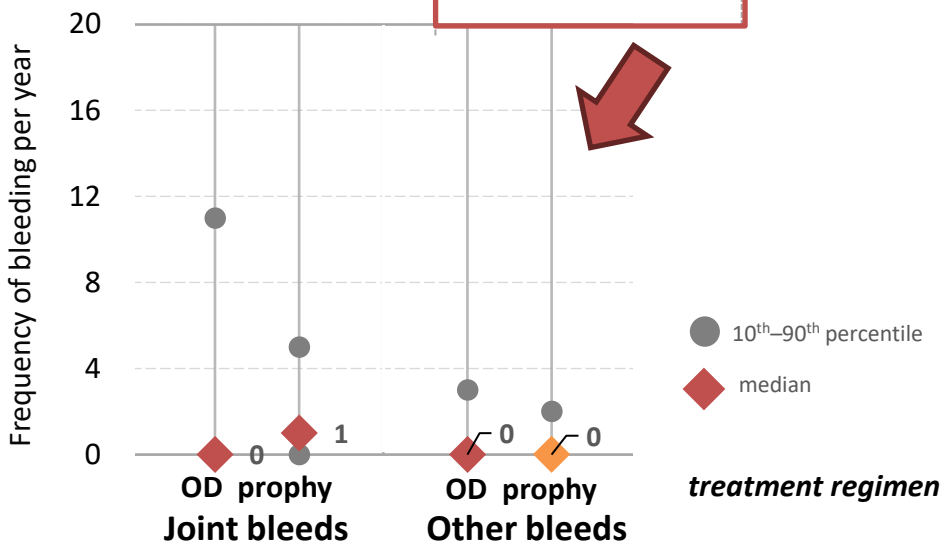
** without inhibitor; missing severity in 6 adults; missing ABR in 5 adults*

Joint and other bleeds according to treatment regimen

Adults
Haem A
N=446*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*251*	*0*	*35*	*4*	*43*	*113*
JOINT BLEEDS						
Mean	*0.1*		*0.7*	*2.5*	***3.8***	***2.3***
Median (range)	*0 (0 – 2)*		*0 (0 – 7)*	*2.5 (2 – 3)*	***0 (0 – 24)***	***1 (0 – 50)***
Total no of recorded bleeds	*15*		*23*	*10*	***164***	***254***
OTHER BLEEDS						
Mean	*0.1*		*0.1*	*1.0*	***1.1***	***0.5***
Median (range)	*0 (0 – 4)*		*0 (0 – 2)*	*1 (0 – 2)*	***0 (0 – 12)***	***0 (0 – 5)***
Total no of recorded bleeds	*18*		*5*	*4*	***48***	***58***

** without inhibitor; missing severity in 6 adults; missing ABR in 5 adults; missing location of bleeds in 4 adults*



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

ABR according to treatment regimen and age

Adults Haem A N=450*

** without inhibitor; missing severity in 6 adults; missing ABR in 5 adults*

Frequency of bleeding	Mild*		Moderate*		Severe*		
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	
N valid	192	0	21	3	38	81	Adults (haem A) born before 1990 N=334
Mean	0.1		0.7	3.7	**8.3**	**3.5**	
Median (min – max)	0 (0 – 4)		0 (0 – 9)	4 (2 – 5)	**4 (0 – 48)**	**1 (0 – 51)**	
Total no of recorded bleeds	20		15	11	**317**	**285**	
adults on permanent prophylaxis	**0 (0%)**		**3 (12.5%)**		**81 (65.9%)**		
% of factor (FVIII) consumed by adults on permanent prophylaxis	-		**79.4%**		**91.6%**		

Frequency of bleeding	Mild*		Moderate*		Severe*		
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	
N valid	60	0	14	1	9	32	Adults (haem A) born in 1990 or later N=116
Mean	0.2		0.9	3.0	**1.2**	**1.6**	
Median (min – max)	0 (0 – 2)		0 (0 – 7)	3 (3 – 3)	**0 (0 – 5)**	**1 (0 – 9)**	
Total no of recorded bleeds	13		13	3	**11**	**52**	
adults on permanent prophylaxis	**0 (0%)**		**1 (6.7%)**		**32 (76.2%)**		
% of factor (FVIII) consumed by adults on permanent prophylaxis	-		**71.1%**		**90.4%**		

Joint and other bleeds according to treatment regimen and age

Adults Haem A N=446*

without inhibitor; missing severity in 6 adults; missing ABR in 5 adults; missing location of bleeds in 4 adults

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	191	0	21	3	34	81
JOINT BLEEDS						
Mean	0.1		0.5	2.3	**4.7**	**2.8**
Median (range)	0 (0 – 2)		0 (0 – 7)	2 (2 – 3)	**1.5 (0 – 24)**	**1 (0 – 50)**
Total no of recorded bleeds	10		11	7	**159**	**221**
OTHER BLEEDS						
Mean	0.1		0.2	1.3	**1.2**	**0.5**
Median (range)	0 (0 – 4)		0 (0 – 2)	2 (0 – 2)	**0 (0 – 12)**	**0 (0 – 4)**
Total no of recorded bleeds	10		4	4	**42**	**39**

Adults (haem A) born before 1990 N=330

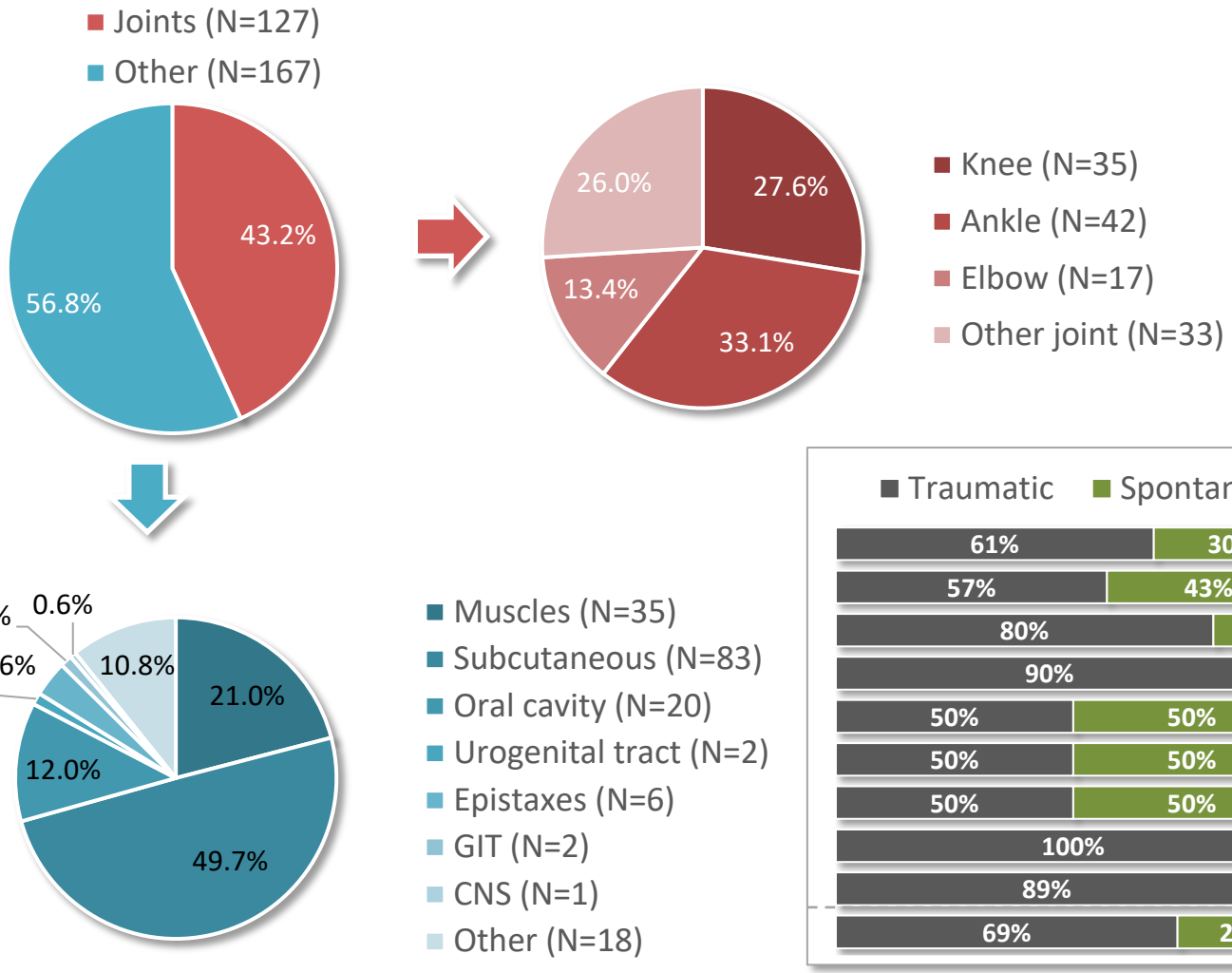
Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	60	0	14	1	9	32
JOINT BLEEDS						
Mean	0.1		0.9	3.0	**0.6**	**1.0**
Median (range)	0 (0 – 1)		0 (0 – 7)	3 (3 – 3)	**0 (0 – 4)**	**0 (0 – 9)**
Total no of recorded bleeds	5		12	3	**5**	**33**
OTHER BLEEDS						
Mean	0.1		0.1	0.0	**0.7**	**0.6**
Median (range)	0 (0 – 2)		0 (0 – 1)	0 (0 – 0)	**0 (0 – 3)**	**0 (0 – 5)**
Total no of recorded bleeds	8		1	0	**6**	**19**

Adults (haem A) born in 1990 or later N=116

Location and etiology of bleeds

Children Haem A N=294*

** number of bleeds*



Detailed treatment of bleeds

Children Haem A N=294*

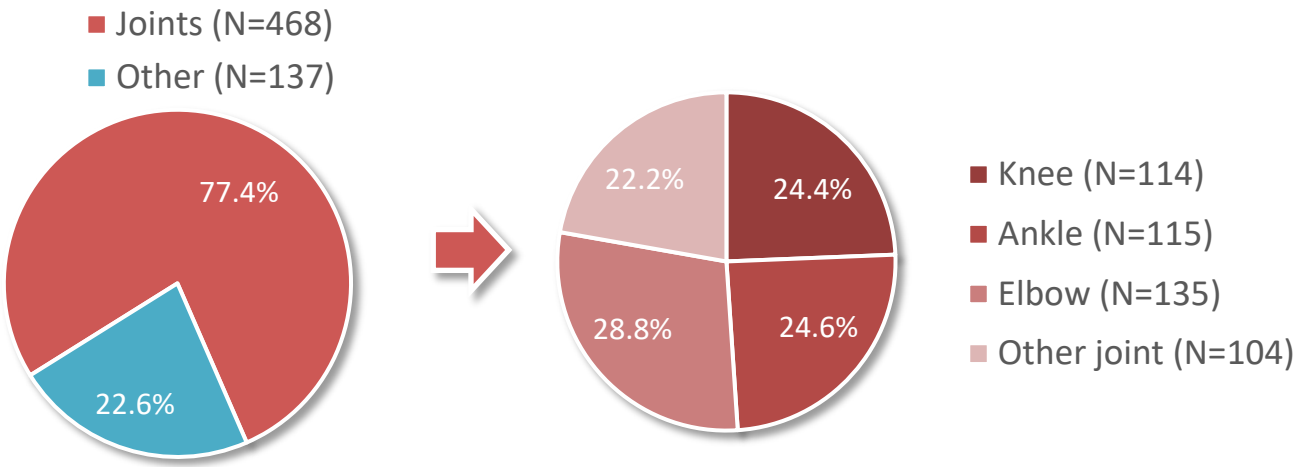
** number of bleeds*

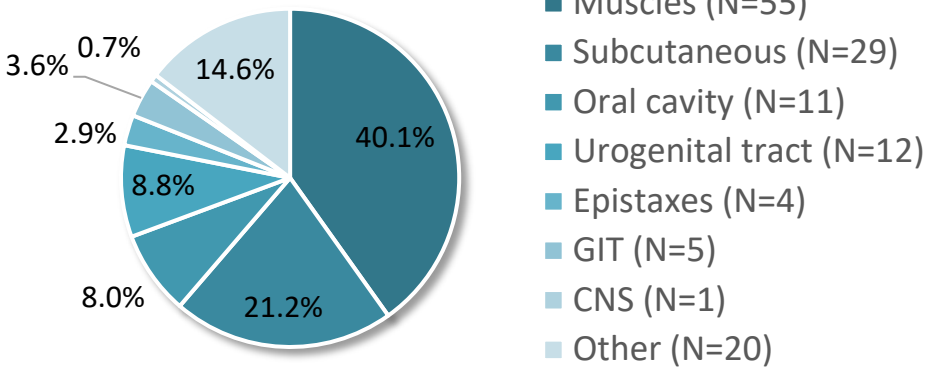
	Joints	Muscles	Subcuta-neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	*127*	*35*	*83*	*20*	*2*	*6*	*2*	*1*	*18*	***294***
FVIII consumption per bleed (IU), valid N	*118*	*32*	*76*	*20*	*2*	*6*	*2*	*1*	*18*	***275***
geometric mean	1542.3	2316.5	916.5	886.1	7348.5	793.7	4582.6	43000.0	964.3	**1326.6**
median	**1500.0**	**2000.0**	**1000.0**	**1000.0**	**7500.0**	**1000.0**	**11000.0**	**43000.0**	**1000.0**	**1000.0**
min – max	500–55000	500–67000	250–4000	250–6000	6000–9000	500–1000	1000–21000	43000–43000	250–4750	**250–67000**
sum	293250	172750	94200	27250	15000	5000	22000	43000	24250	**696700**
No. of doses per bleed										
geometric mean	1.4	2.1	1.3	1.3	4.5	1.0	2.6	30.0	1.3	**1.4**
median	**1**	**2**	**1**	**1**	**5**	**1**	**4**	**30**	**1**	**1**
min – max	1–27	1–30	1–6	1–11	4–5	1–1	1–7	30–30	1–12	**1–30**
Duration of therapy per bleed, days										
geometric mean	1.5	2.3	1.5	1.4	2.8	1.0	2.0	14.0	1.6	**1.6**
median	**1**	**2**	**1**	**1**	**3**	**1**	**3**	**14**	**1**	**1**
min – max	1–14	1–25	1–7	1–13	2–4	1–1	1–4	14–14	1–16	**1–25**
N (%) with hospitalization	*6 (4.7%)*	*2 (5.7%)*	*7 (8.4%)*	*3 (15%)*	*1 (50%)*	*0 (0%)*	*1 (50%)*	*1 (100%)*	*1 (5.6%)*	***22 (7.5%)***
N (%) with rebleeding	*0 (0%)*	*0 (0%)*	*2 (2.4%)*	*1 (5%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*	*1 (5.6%)*	***4 (1.4%)***

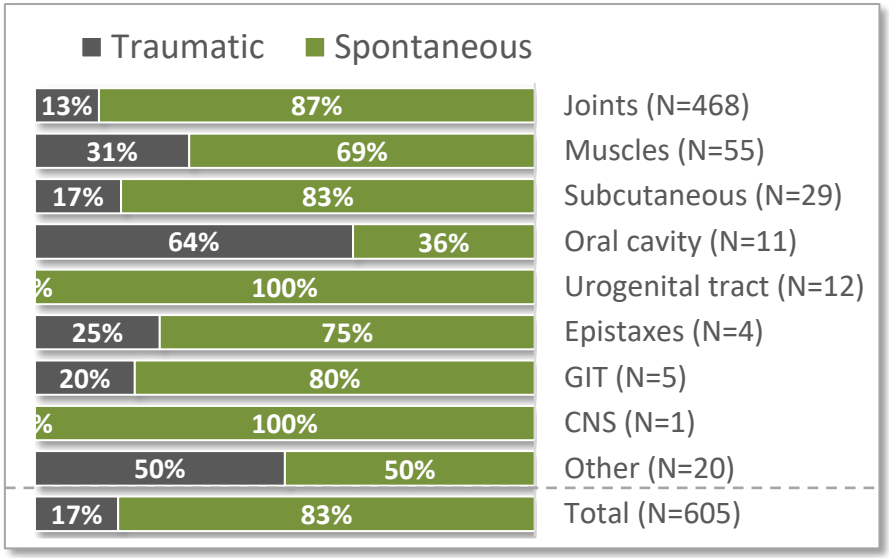
Location and etiology of bleeds

Adults
Haem A
N=605*

** number of bleeds*







Detailed treatment of bleeds

Adults
Haem A
N=605*

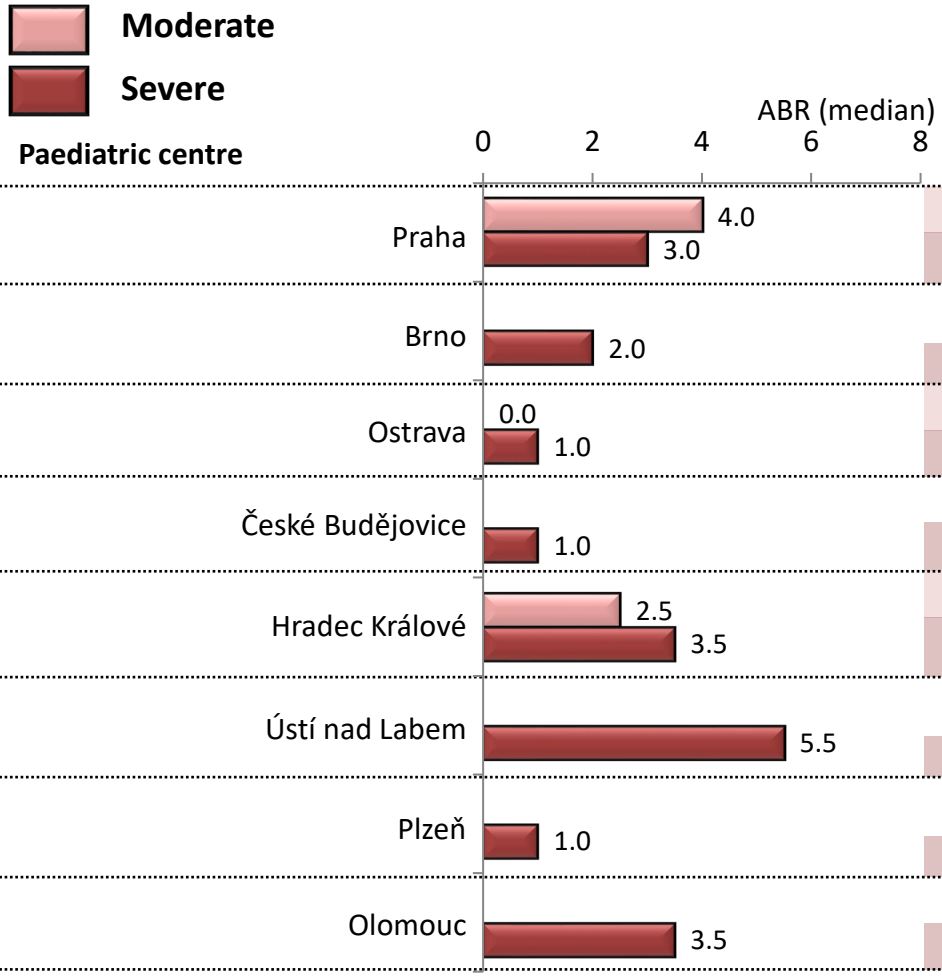
** number of bleeds*

	Joints	Muscles	Subcuta-neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	*468*	*55*	*29*	*11*	*12*	*4*	*5*	*1*	*20*	***605***
FVIII consumption per bleed (IU), valid N	*466*	*52*	*29*	*11*	*11*	*4*	*5*	*1*	*20*	***599***
geometric mean	2141.8	2804.8	1928.4	2716.3	3051.8	1565.1	7344.8	31500.0	2744.0	**2251.9**
median	**2000.0**	**3000.0**	**2000.0**	**2500.0**	**5000.0**	**1750.0**	**15000.0**	**31500.0**	**2000.0**	**2000.0**
min – max	250–60000	500–52000	500–15000	500–22000	500–11000	1000–2000	1000–50000	31500–31500	500–51000	**250–60000**
sum	1605011	272000	102000	60500	55000	6500	86500	31500	120500	**2339511**
No. of doses per bleed										
geometric mean	1.7	2.5	1.7	1.7	2.2	1.0	6.1	58.0	2.0	**1.8**
median	**1**	**2**	**1**	**1**	**2**	**1**	**7**	**58**	**2**	**1**
min – max	1–58	1–32	1–14	1–11	1–8	1–1	1–53	58–58	1–21	**1–58**
Duration of therapy per bleed, days										
geometric mean	1.5	2.1	1.8	1.6	2.4	1.0	5.8	43.0	1.6	**1.6**
median	**1**	**2**	**2**	**1**	**2**	**1**	**6**	**43**	**1**	**1**
min – max	1–28	1–25	1–12	1–11	1–8	1–1	1–39	43–43	1–10	**1–43**
N (%) with hospitalization	*9 (1.9%)*	*4 (7.3%)*	*0 (0%)*	*1 (9.1%)*	*1 (8.3%)*	*0 (0%)*	*3 (60%)*	*1 (100%)*	*1 (5%)*	***20 (3.3%)***
N (%) with rebleeding	*23 (4.9%)*	*2 (3.6%)*	*0 (0%)*	*1 (9.1%)*	*1 (8.3%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*	***27 (4.5%)***

ABR according to centres Haemophilia A (PWHA)

Annual bleeding rate on permanent prophylaxis

HaemA on prophy
Paed. centres
N=84

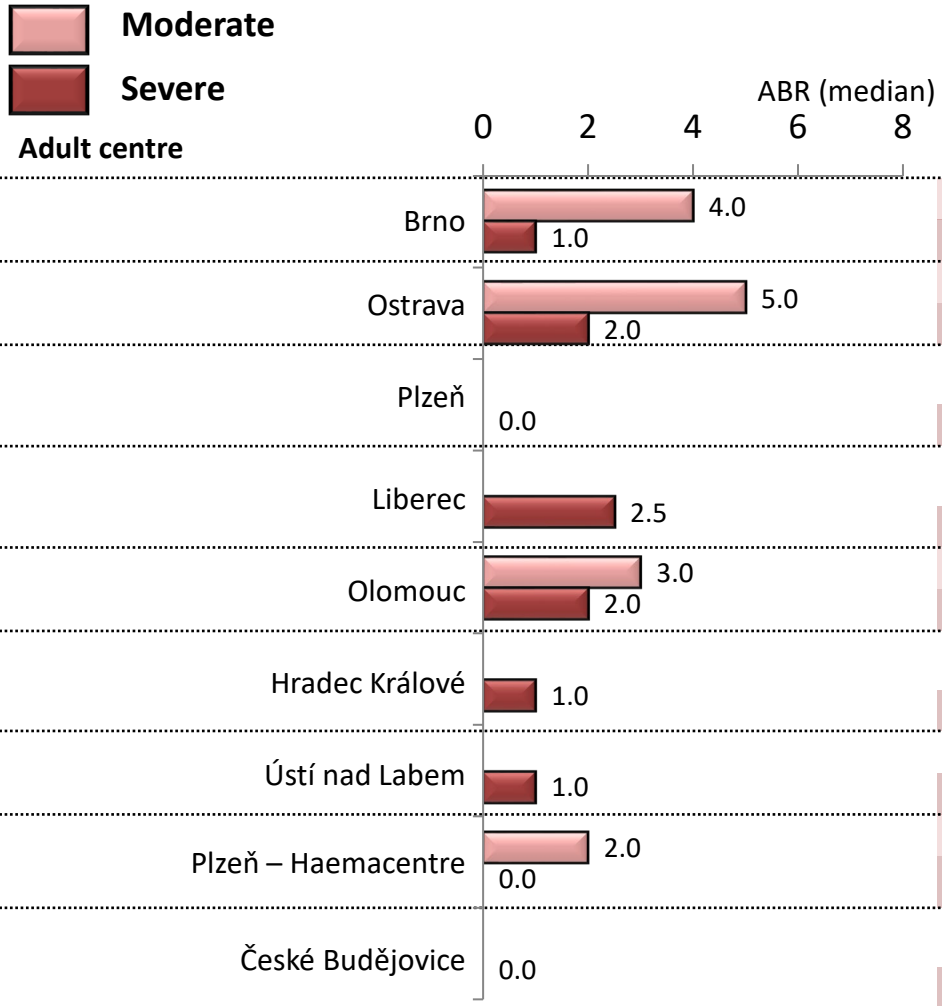


Frequency of bleeding in PWHA without inhibitor on permanent prophylaxis

Paediatric centre	N	Mean	Median	Min	Max	Severity
Praha	2	4.0	4.0	3	5	Moderate
	32	3.3	3.0	0	10	Severe
Brno	0					
	15	2.3	2.0	0	8	Severe
Ostrava	4	0.5	0.0	0	2	Moderate
	8	0.8	1.0	0	2	Severe
České Budějovice	0					
	12	2.1	1.0	0	9	Severe
Hradec Králové	2	2.5	2.5	0	5	Moderate
	2	3.5	3.5	3	4	Severe
Ústí nad Labem	0					
	2	5.5	5.5	0	11	Severe
Plzeň	0					
	3	1.3	1.0	0	3	Severe
Olomouc	0					
	2	3.5	3.5	3	4	Severe

Annual bleeding rate on permanent prophylaxis

HaemA on prophy
Adult centres
N=106

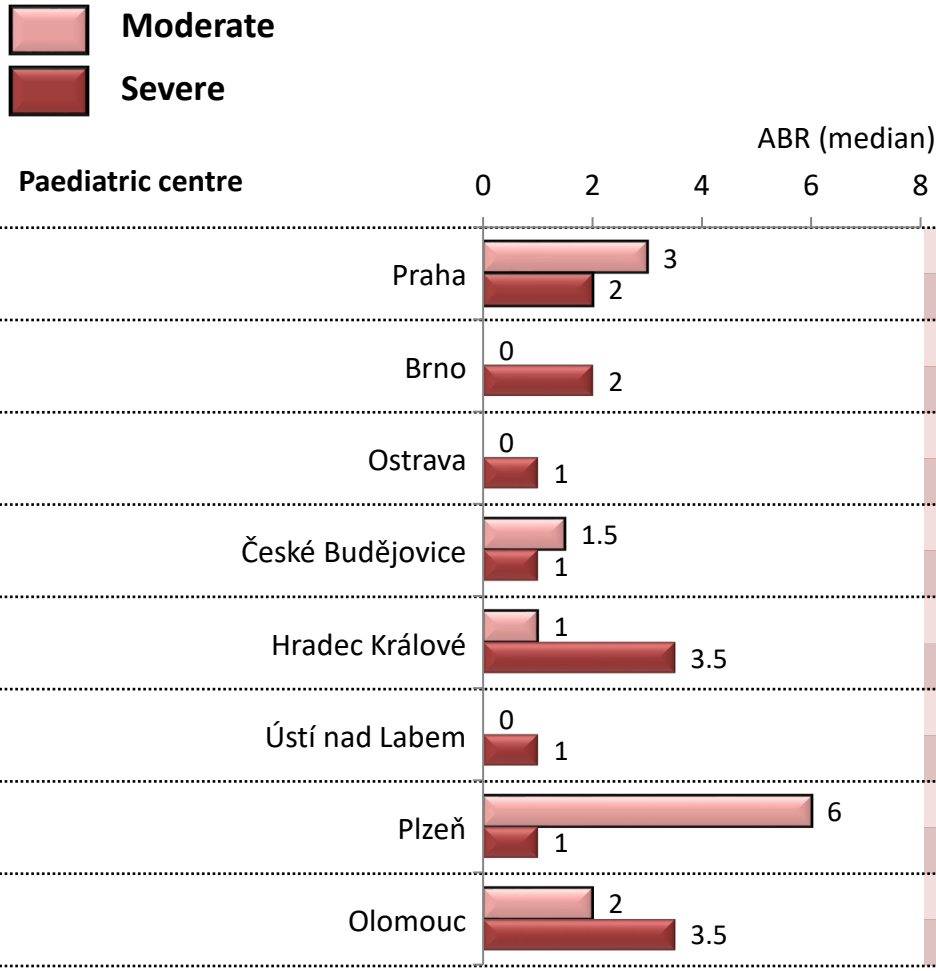


Frequency of bleeding in PWHA without inhibitor on permanent prophylaxis

Adult centre	N	Mean	Median	Min	Max	Severity
Brno	1	4.0	4.0	4	4	Moderate
	27	1.7	1.0	0	12	Severe
Ostrava	1	5.0	5.0	5	5	Moderate
	22	2.3	2.0	0	9	Severe
Plzeň	0					
	12	3.3	0.0	0	26	Severe
Liberec	0					
	6	2.8	2.5	0	5	Severe
Olomouc	1	3.0	3.0	3	3	Moderate
	9	5.0	2.0	0	20	Severe
Hradec Králové	0					
	9	8.3	1.0	0	51	Severe
Ústí nad Labem	0					
	7	3.4	1.0	0	9	Severe
Plzeň – Haemacentre	1	2.0	2.0	2	2	Moderate
	3	0.0	0.0	0	0	Severe
České Budějovice	0					
	7	2.4	0.0	0	12	Severe

Annual bleeding rate regardless prophylaxis

HaemA
Paed. centres
N=117



Frequency of bleeding in PWHA without inhibitor regardless of prophylaxis

Paediatric centre	N	Mean	Median	Min	Max	% on permanent prophylaxis
Praha	8	3.0	3.0	0	6	25.0%
	38	3.0	2.0	0	10	84.2%
Brno	4	0.0	0.0	0	0	0.0%
	19	2.0	2.0	0	8	78.9%
Ostrava	5	1.8	0.0	0	7	80.0%
	8	0.8	1.0	0	2	100.0%
České Budějovice	2	1.5	1.5	1	2	0.0%
	13	2.0	1.0	0	9	92.3%
Hradec Králové	3	2.0	1.0	0	5	66.7%
	2	3.5	3.5	3	4	100.0%
Ústí nad Labem	2	0.0	0.0	0	0	0.0%
	3	4.0	1.0	0	11	66.7%
Plzeň	1	6.0	6.0	6	6	0.0%
	5	1.6	1.0	0	4	60.0%
Olomouc	2	2.0	2.0	0	4	0.0%
	2	3.5	3.5	3	4	100.0%

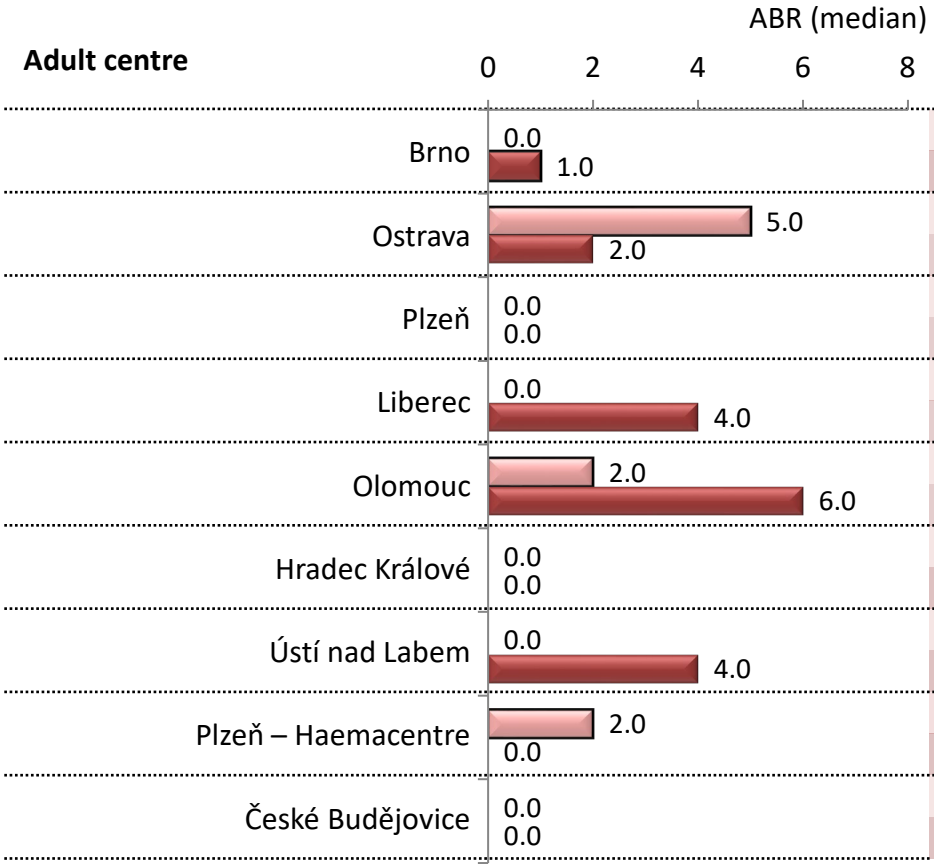
Annual bleeding rate regardless prophylaxis

HaemA
Adult centres
N=183*

** missing ABR in 5 adults*

Moderate
Severe

Frequency of bleeding in PWHA without inhibitor regardless of prophylaxis



Adult centre	N*	Mean	Median	Min	Max	% on permanent prophylaxis
Brno	13	0.5	0.0	0	4	7.7%
	37	1.5	1.0	0	12	73.0%
Ostrava	5	3.8	5.0	0	9	20.0%
	24	2.3	2.0	0	9	91.7%
Plzeň	3	0.0	0.0	0	0	0.0%
	19	8.2	0.0	0	48	60.0%
Liberec	2	0.0	0.0	0	0	0.0%
	10	6.7	4.0	0	24	60.0%
Olomouc	2	2.0	2.0	1	3	50.0%
	20	7.4	6.0	0	20	45.0%
Hradec Králové	5	0.2	0.0	0	1	0.0%
	13	7.8	0.0	0	51	52.9%
Ústí nad Labem	3	0.0	0.0	0	0	0.0%
	8	4.0	4.0	0	9	87.5%
Plzeň – Haemacentre	1	2.0	2.0	2	2	100.0%
	3	0.0	0.0	0	0	100.0%
České Budějovice	4	1.8	0.0	0	7	0.0%
	11	1.5	0.0	0	12	63.6%

Prophylactic regimens and treatment outcomes

HaemA
Paed. centres
N=117

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	50.9	**50.9**	45.5	56.4	52.6	**52.6**	6	6.0	8.0
	Severe	38	**84.2%**	32	76.1	**74.6**	33.3	125.0	56.1	**113.3**	6	6.0	2.0
Brno	**Moderate**	4	**0.0%**	0							4	4.0	2.0
	Severe	19	**78.9%**	15	84.3	**75.4**	27.8	160.4	19.3	**19.3**	4	4.0	2.0
Ostrava	**Moderate**	5	**80.0%**	4	53.2	**54.1**	43.5	61.0	0.0	**0.0**	1	1.0	0.0
	Severe	8	**100.0%**	8	90.7	**87.8**	66.0	121.7	0.0	**0.0**	0		
Č. Budějovice	**Moderate**	2	**0.0%**	0							2	2.0	1.0
	Severe	13	**92.3%**	12	60.2	**62.0**	24.3	109.2	0.0	**0.0**	1	1.0	0.0
Hradec Králové	**Moderate**	3	**66.7%**	2	44.8	**44.8**	12.7	76.9	0.0	**0.0**	1	1.0	1.0
	Severe	2	**100.0%**	2	88.0	**88.0**	68.4	107.5	0.0	**0.0**	0		
Ústí nad Labem	**Moderate**	2	**0.0%**	0							2	2.0	0.0
	Severe	3	**66.7%**	2	87.3	**87.3**	75.0	99.6	71.4	**71.4**	1	1.0	1.0
Plzeň	**Moderate**	1	**0.0%**	0							1	1.0	1.0
	Severe	5	**60.0%**	3	65.6	**59.3**	56.2	81.2	31.3	**31.3**	2	2.0	1.0
Olomouc	**Moderate**	2	**0.0%**	0							2	2.0	1.0
	Severe	2	**100.0%**	2	47.8	**47.8**	38.3	57.3	0.0	**0.0**	0		

Prophylactic regimens and treatment outcomes

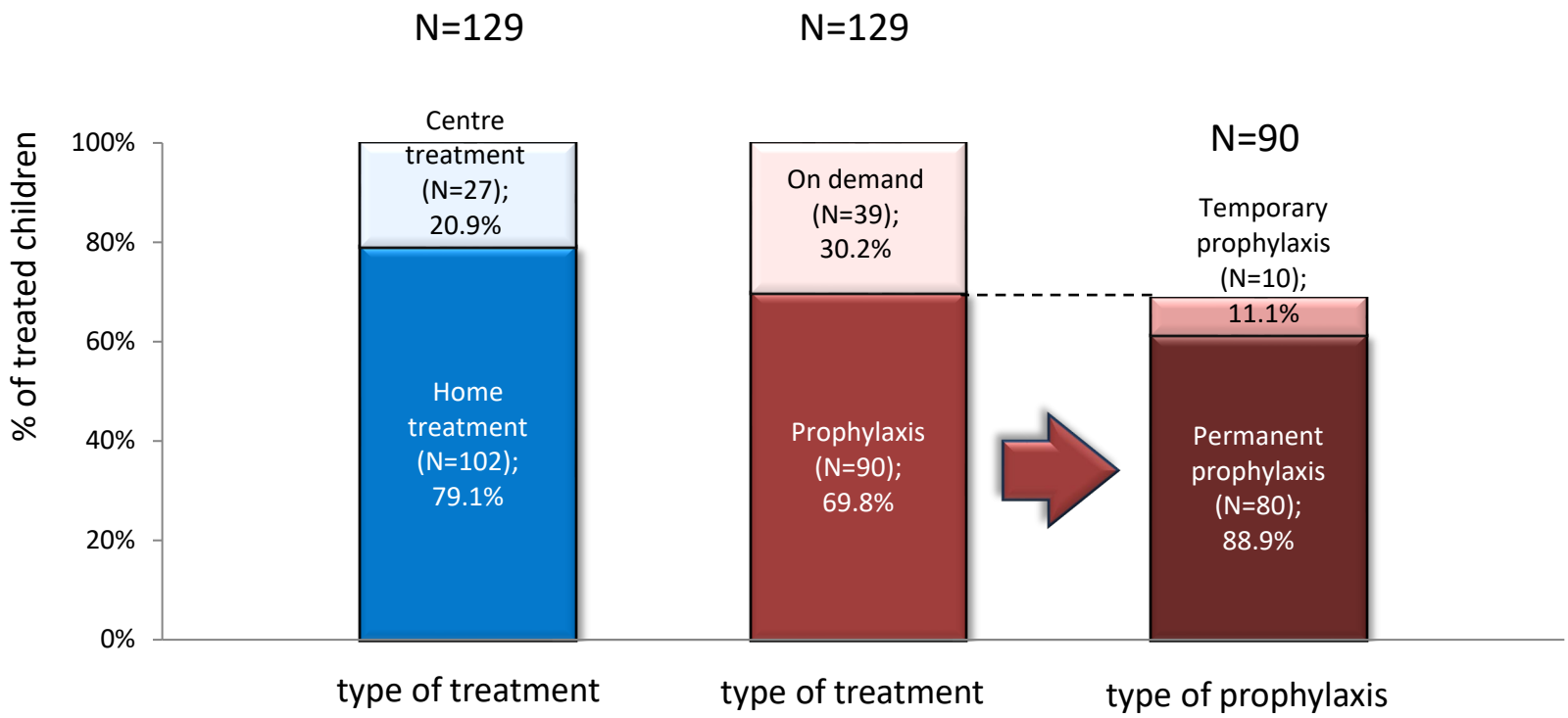
HaemA
Adult centres
N=188

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	13	**7.7%**	1	42.9	**42.9**	42.9	42.9	4.0	**4.0**	30	12	0.2	0.0	47
	Severe	37	**73.0%**	27	57.1	**51.9**	21.9	121.6	1.7	**1.0**	38	10	1.2	0.0	47
Ostrava	Moderate	5	**20.0%**	1	70.2	**70.2**	70.2	70.2	5.0	**5.0**	67	4	3.5	2.5	54
	Severe	24	**91.7%**	22	60.0	**57.9**	31.4	112.9	2.3	**2.0**	40	2	2.5	2.5	68
Plzeň	Moderate	3	**0.0%**	0								3	0.0	0.0	43
	Severe	20	**60.0%**	12	43.7	**40.6**	13.3	111.9	3.3	**0.0**	46	8	16.6	12.0	55
Liberec	Moderate	2	**0.0%**	0								2	0.0	0.0	47
	Severe	10	**60.0%**	6	59.3	**60.2**	26.8	100.0	2.8	**2.5**	38	4	12.5	11.5	65
Olomouc	Moderate	2	**50.0%**	1	77.9	**77.9**	77.9	77.9	3.0	**3.0**	20	1	1.0	1.0	24
	Severe	20	**45.0%**	9	51.9	**60.1**	23.3	85.7	5.0	**2.0**	31	11	9.3	11.0	59
Hradec Králové	Moderate	5	**0.0%**	0								5	0.2	0.0	24
	Severe	17	**52.9%**	9	66.5	**60.6**	45.5	104.7	8.3	**1.0**	33	8	6.8	0.0	35
Ústí n. Labem	Moderate	3	**0.0%**	0								3	0.0	0.0	22
	Severe	8	**87.5%**	7	43.2	**40.4**	31.4	64.0	3.4	**1.0**	35	1	8.0	8.0	44
Plzeň - Haemacentre	Moderate	1	**100.0%**	1	28.8	**28.8**	28.8	28.8	2.0	**2.0**	50	0			
	Severe	3	**100.0%**	3	60.4	**73.2**	23.8	84.3	0.0	**0.0**	46	0			
Č. Budějovice	Moderate	4	**0.0%**	0								4	1.8	0.0	70
	Severe	11	**63.6%**	7	56.0	**53.3**	32.6	94.3	2.4	**0.0**	51	4	0.0	0.0	51

** missing ABR in 5 adults*

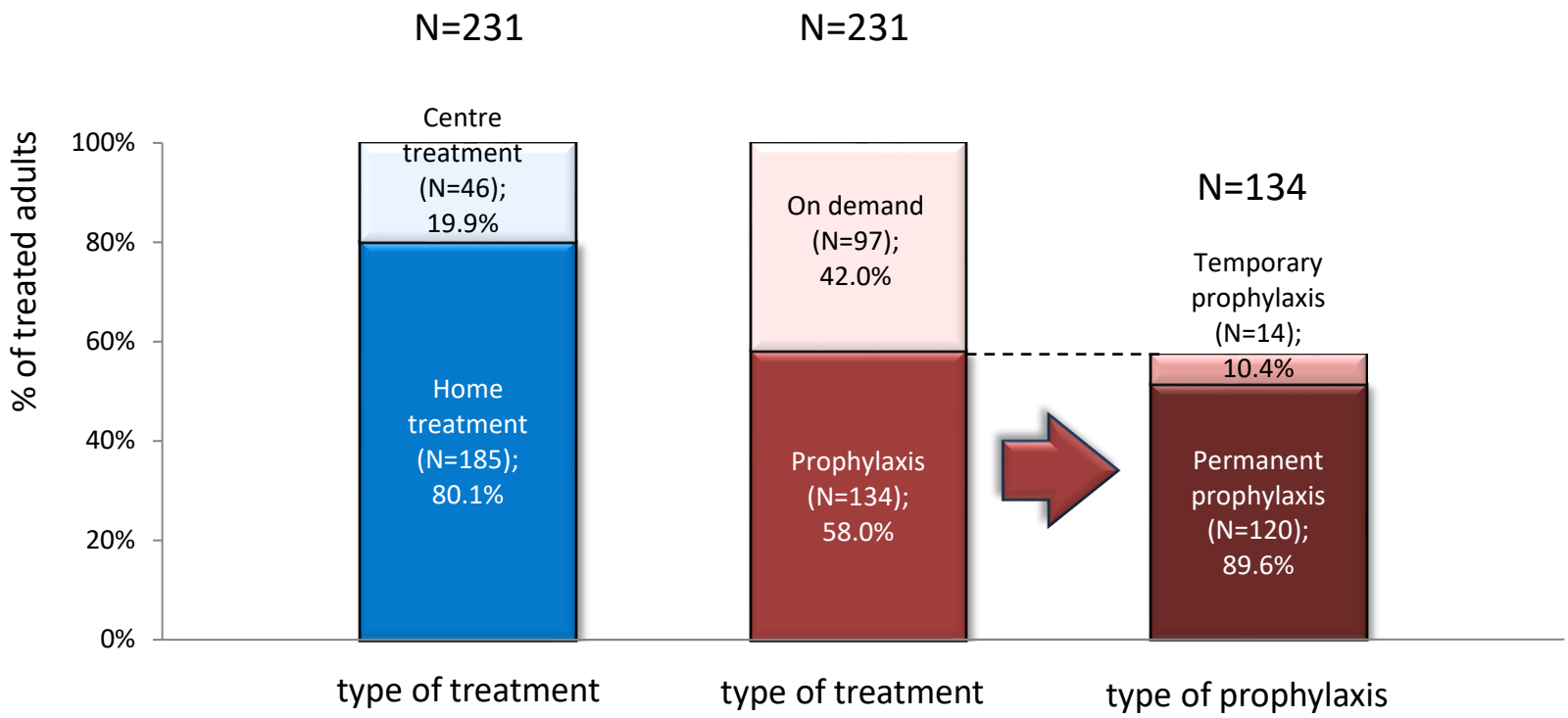
Children
Haem A
N=129

Type of treatment (subgroup of treated patients)



Adults
Haem A
N=231

Type of treatment (subgroup of treated patients)



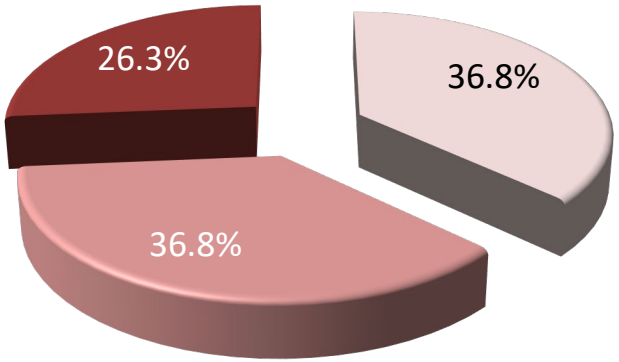
Demographic characteristics Haemophilia B

Haem B
N=105

Severity of haemophilia B

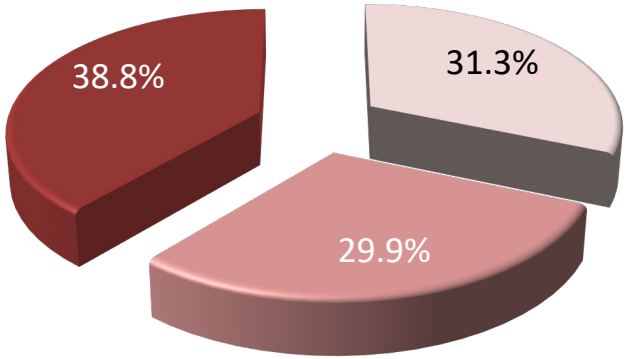
Children (N=38)

- Mild (N=14)
- Moderate (N=14)
- Severe (N=10)



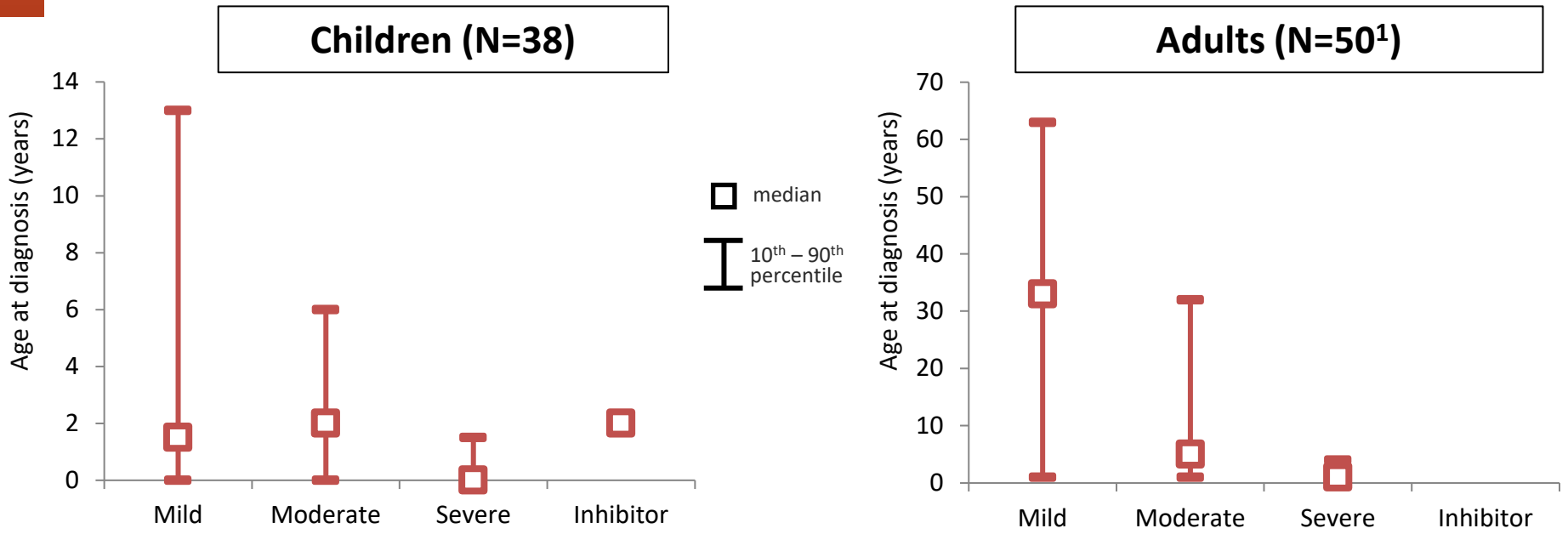
Adults (N=67)

- Mild (N=21)
- Moderate (N=20)
- Severe (N=26)



Age at diagnosis according to severity of haemophilia B

Haem B
N=105



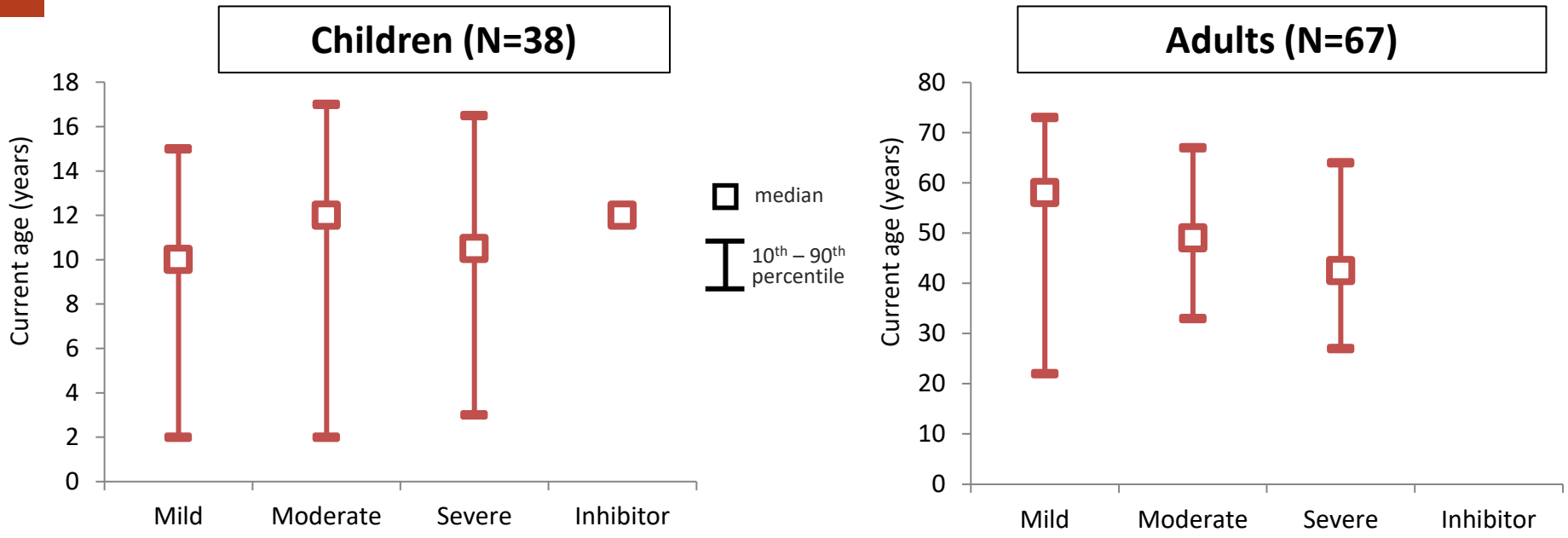
Mild*	Moderate*	Severe*	Inhibitor+	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor+
14	*14*	*10*	*1*	**N valid**	*14*	*15*	*21*	*0*
3.9	*2.5*	*0.5*	*2.0*	**Mean**	*31.2*	*10.0*	*1.7*	
1.5 (0 – 15)	*2 (0 – 8)*	*0 (0 – 2)*	*2 (2 – 2)*	**Median (min – max)**	*33 (0 – 67)*	*5 (0 – 61)*	*1 (0 – 8)*	

[1] *Missing information on year of diagnosis in 17 adults.*

** including persons with inhibitor*
+ in 2019

Haem B
N=105

Actual age according to severity of haemophilia B



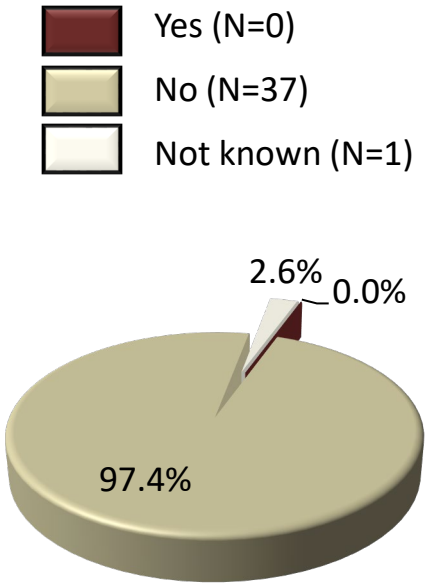
Mild*	Moderate*	Severe*	Inhibitor+	Current age++ (years)	Mild*	Moderate*	Severe*	Inhibitor+
14	*14*	*10*	*1*	**N valid**	*21*	*20*	*26*	*0*
9.0	*10.4*	*9.8*	*12.0*	**Mean**	*51.2*	*49.2*	*43.8*	
10 (1 – 18)	*12 (0 – 18)*	*10.5 (2 – 17)*	*12 (12 – 12)*	**Median (min – max)**	*58 (19 – 94)*	*49 (20 – 70)*	*42.5 (24 – 68)*	

** including persons with inhibitor*
+ in 2019
++ age reached in year 2019

Hepatitis (ever) experienced

Children
Haem B
N=38

Experienced hepatitis



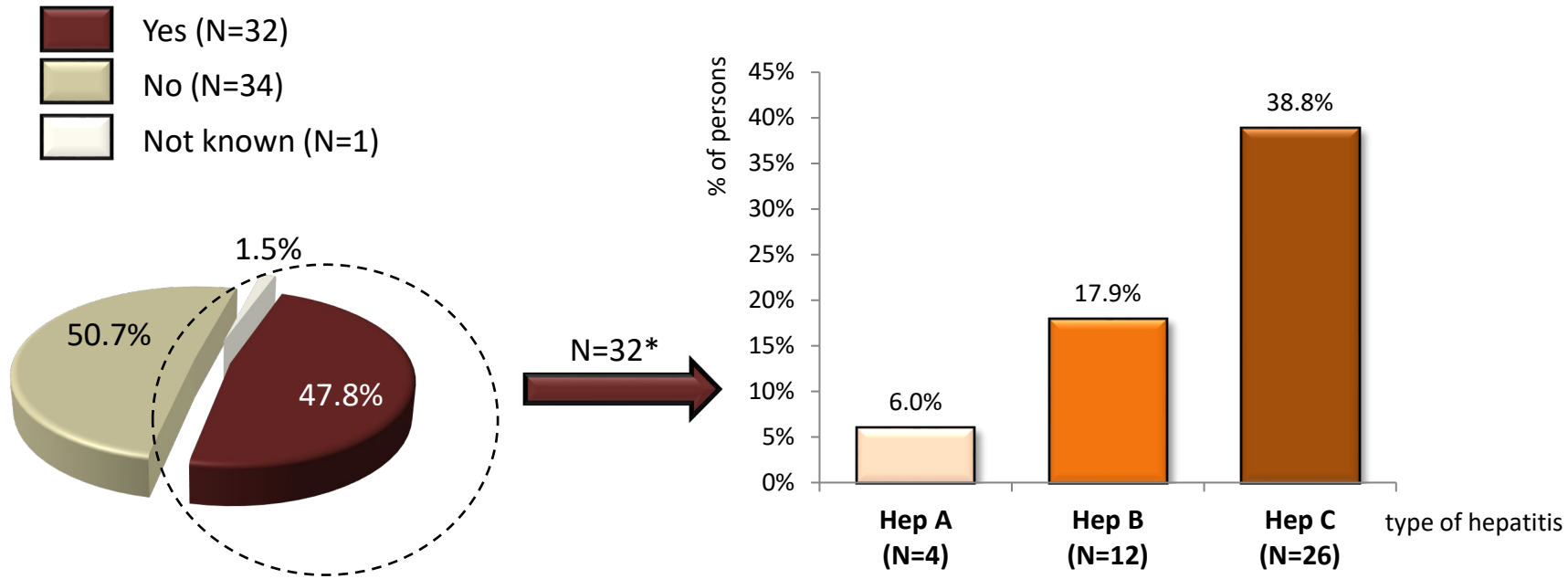
No child has hepatitis C.

Data from last completed annual report of each person.

Hepatitis (ever) experienced

Adults
Haem B
N=67

Experienced hepatitis



Data from last completed annual report of each person.

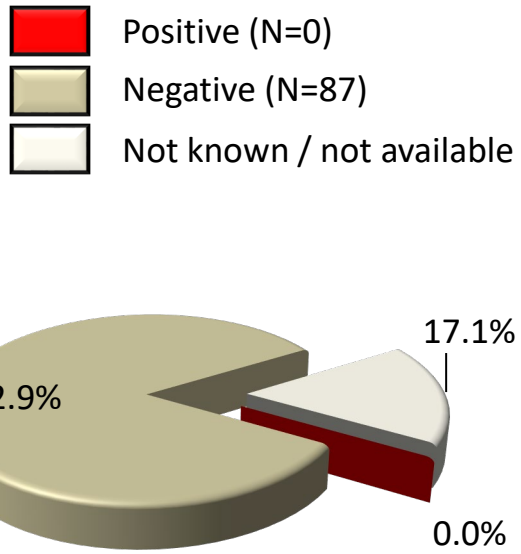
**Total of 42 cases of hepatitis in 32 persons. One person may have more types of hepatitis recorded.*

9 adults are HCV RNA positive

HIV

All
Haem B
N=105

HIV



No HIV-positive person.

Data from last completed annual report of each person.

Treatment outcomes and bleeding frequency Haemophilia B

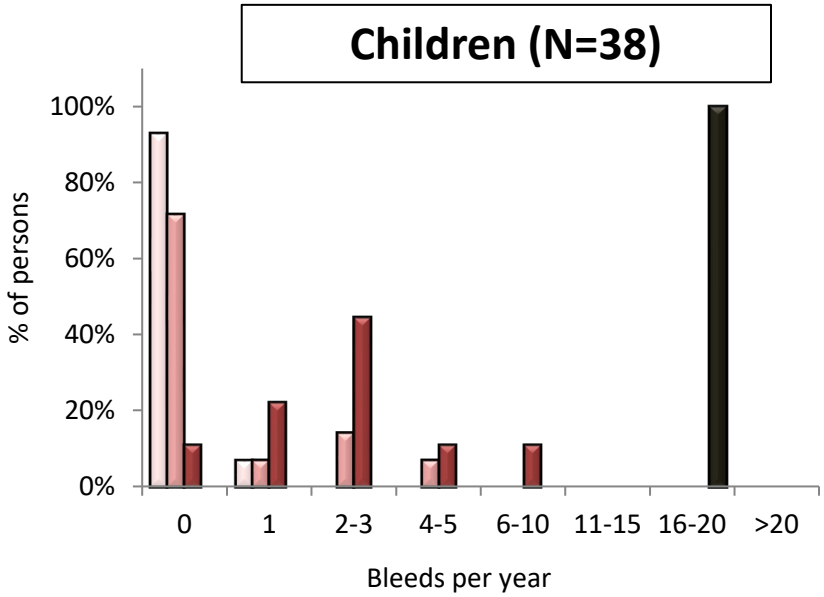
All
Haem B
N=105

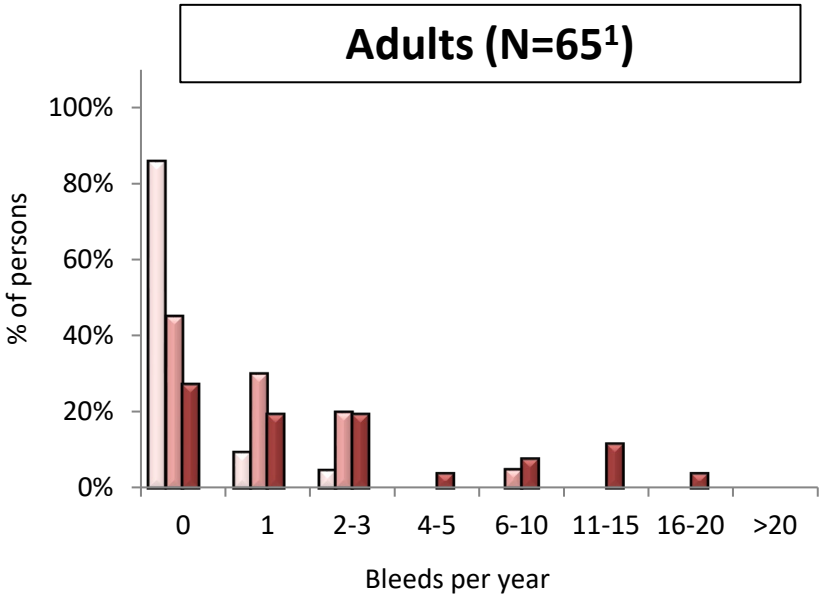
Data from year 2019 – sample size

	Valid persons			Persons with valid annual report			Persons examined			Persons treated	
	N	%		N	%		N	%		N	%
All	105	100%	→	95	90.5%	→	82	78.1%	→	63	60.0%
of them with inhibitor	1			1			1			1	
Children	38	100%	→	37	97.4%	→	32	84.2%	→	19	50.0%
of them with inhibitor	1			1			1			1	
Adults	67	100%	→	58	86.6%	→	50	74.6%	→	44	65.7%
of them with inhibitor	0			0			0			0	

Haem B
N=103[1]

Frequency of bleeding requiring treatment in 2019





Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
14	*14*	*9*	*1*	**N valid**	*21*	*20*	*24*	*0*
0.1	*0.6*	*3.1*	*20.0*	**Mean**	*0.2*	*1.2*	*3.8*	
0 (0 – 1)	*0 (0 – 4)*	*3 (0 – 10)*	*20 (20 – 20)*	**Median (min – max)**	*0 (0 – 2)*	*1 (0 – 8)*	*1.5 (0 – 20)*	
13 (92.9%)	*10 (71.4%)*	*1 (11.1%)*	*0 (0%)*	**N (%) with no bleed**	*18 (85.7%)*	*9 (45%)*	*7 (26.9%)*	

** without inhibitor*

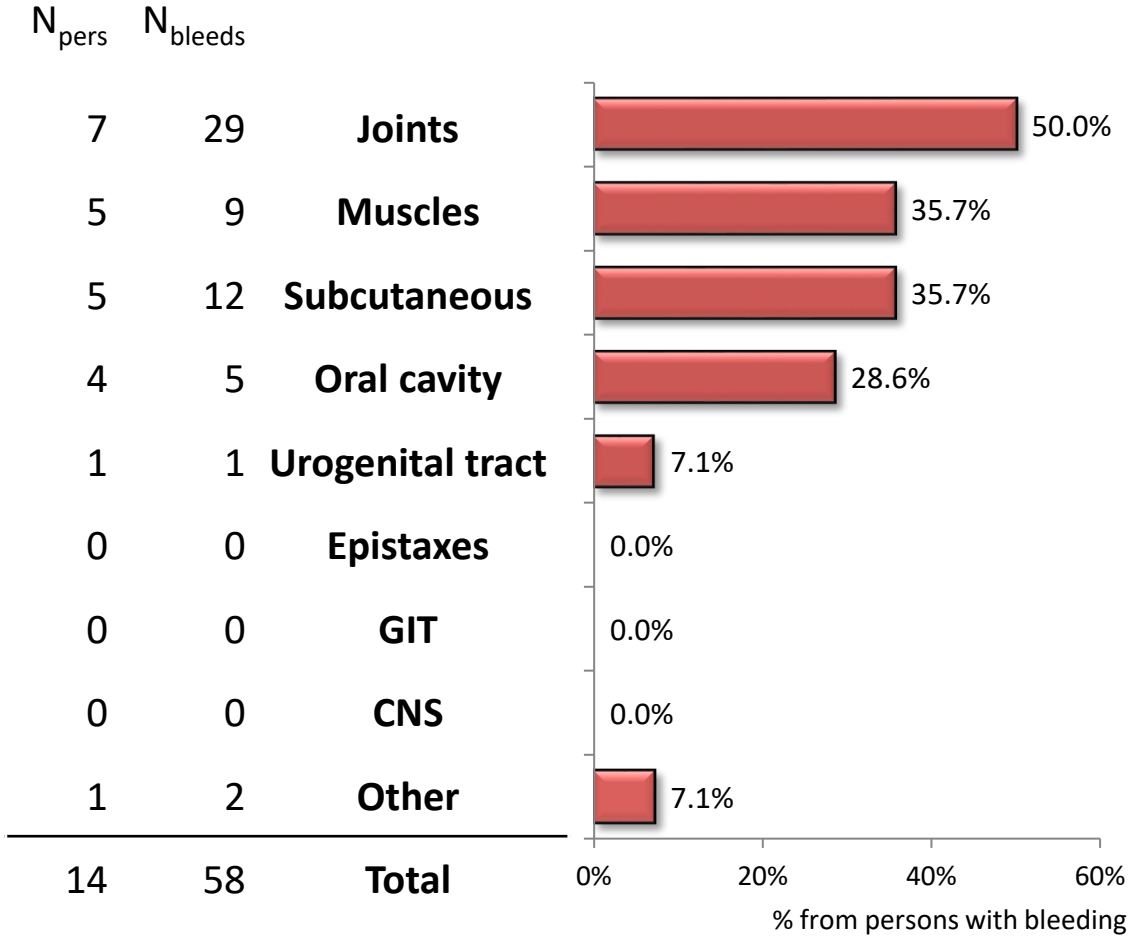
[1]Frequency of bleeding is missing in 2 adults.

Location of bleeds in 2019

Children
Haem B
N=38

14 (36.8%) children experienced bleeding requiring treatment at least once in year; 58 bleeds were recorded in total, 2 bleeds required hospitalization.
All of these children have recorded location of their bleeds.

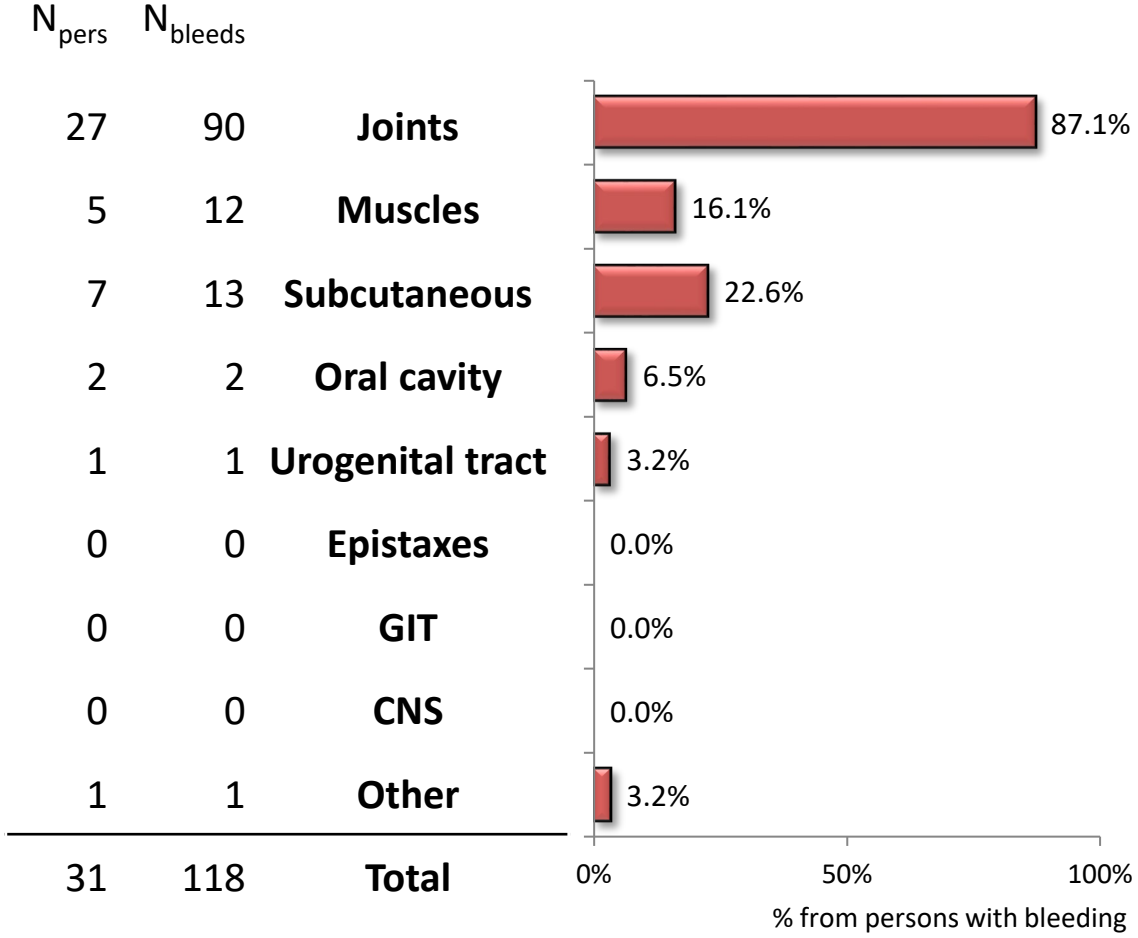
24 (63.2%) children recorded no bleed during year 2019.



Location of bleeds in 2019

Adults
Haem B
N=65[1]

31 (47.7%) adults experienced bleeding requiring treatment at least once in year; 119 bleeds were recorded in total, 1 bleed required hospitalization.
All of these 31 adults have recorded location of their bleeds except of one bleed.
34 (52.3%) adults have recorded no bleed during year 2019.

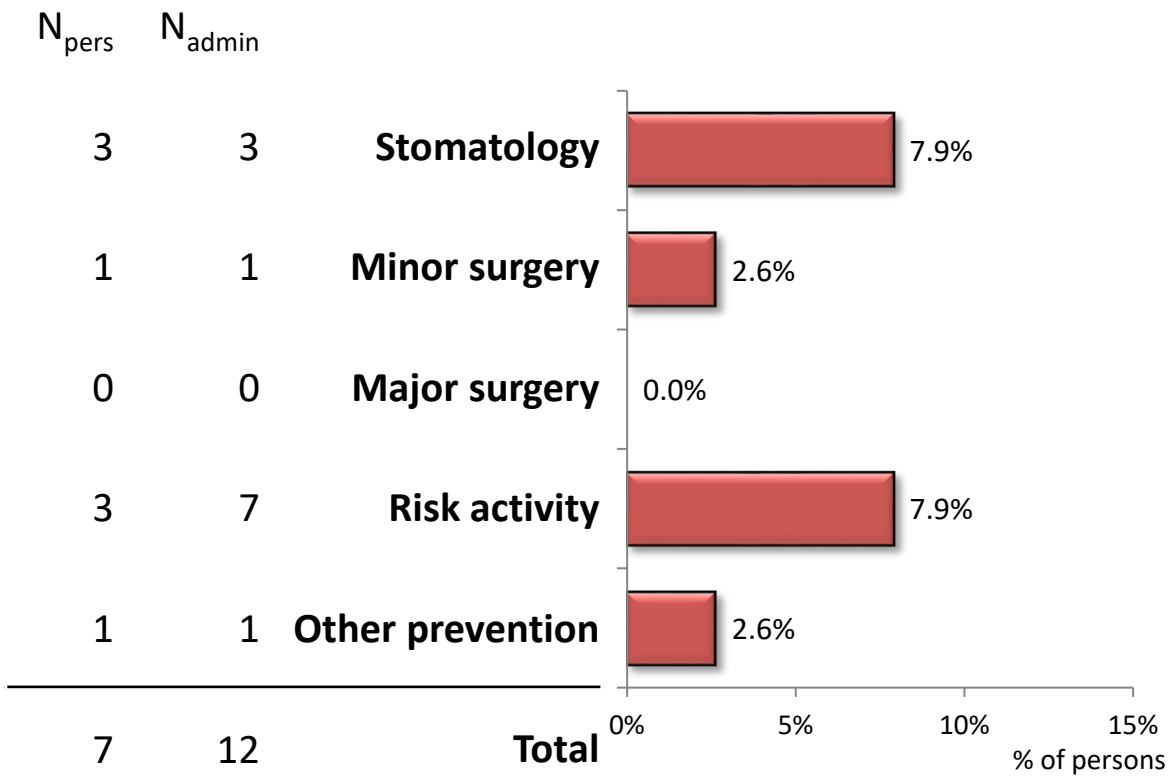


[1]*Frequency of bleeding is missing in 2 adults.*

Preventive administration in 2019

Children
Haem B
N=38

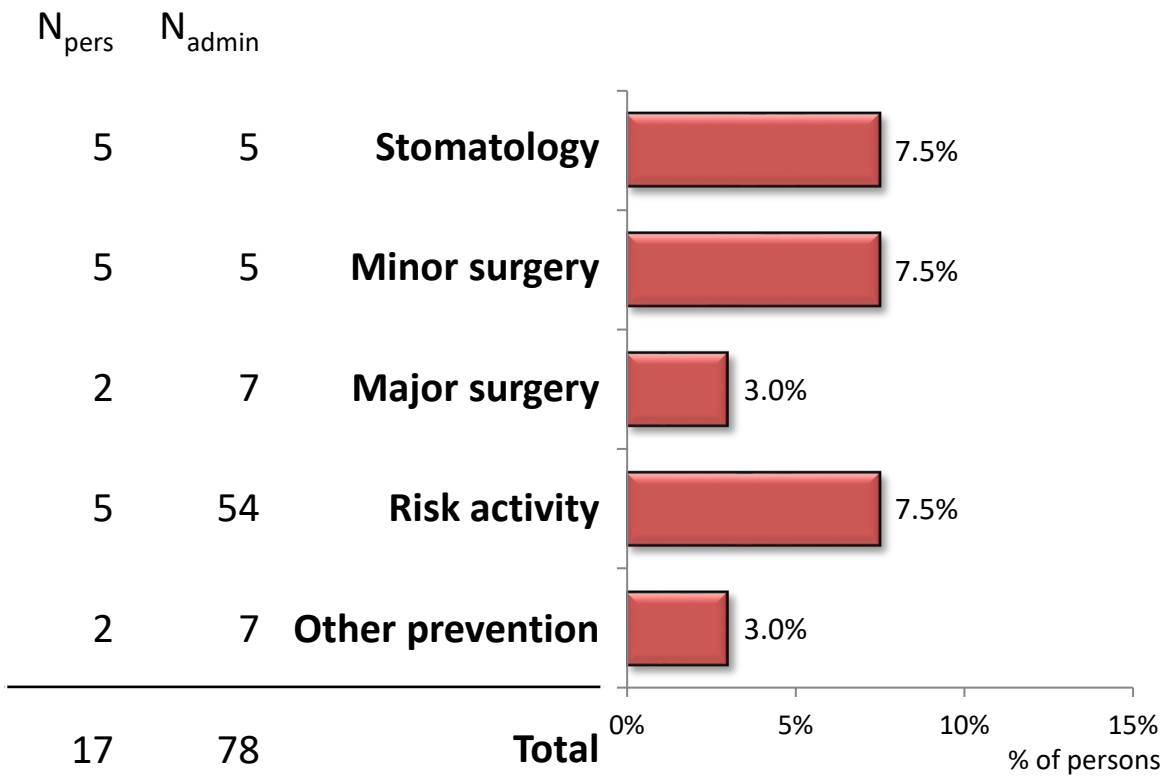
7 (18.4%) children were given factor to prevent bleeding during/before risk situation.
12 preventive administrations were recorded in total.



Preventive administration in 2019

Adults
Haem B
N=67

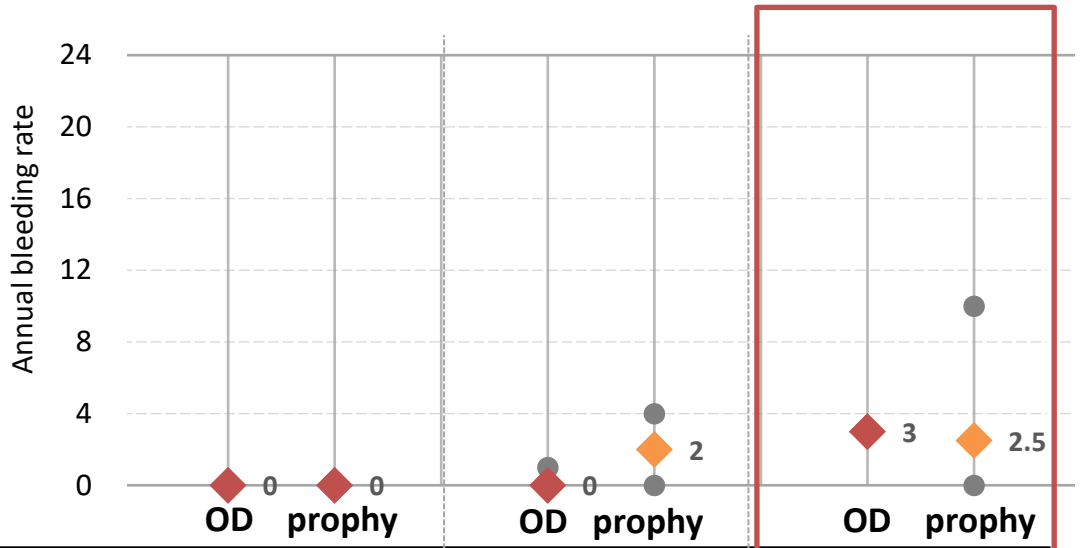
17 (25.4%) persons were given factor to prevent bleeding during/before risk situation.
78 preventive administrations were recorded in total.



ABR according to treatment regimen Haemophilia B without inhibitor

Annual bleeding rate according to treatment regimen

Children Haem B N=37*



Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	14	0	11	3	1	8
Mean	0.1		0.3	2.0	3.0	3.1
Median (min – max)	0 (0 – 1)		0 (0 – 2)	2 (0 – 4)	3 (3 – 3)	2.5 (0 – 10)
Total no of recorded bleeds	1		3	6	3	25
children on permanent prophylaxis	0 (0%)		3 (21.4%)		8 (88.9%)	
% of factor (FIX) consumed by children on permanent prophylaxis	-		98.4%		99.8%	

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

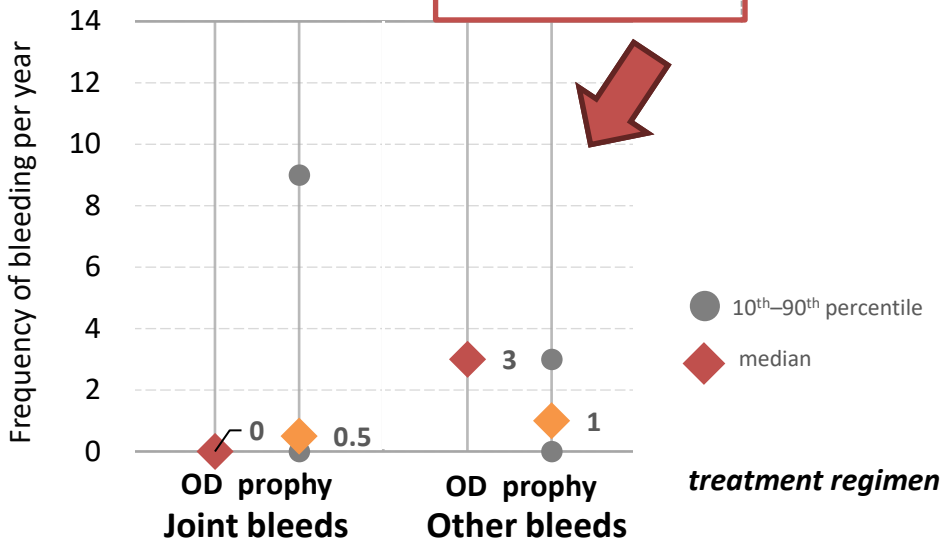
** without inhibitor*

Joint and other bleeds according to treatment regimen

Children Haem B N=37*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*14*	*0*	*11*	*3*	*1*	*8*
JOINT BLEEDS						
Mean	*0.0*	0	*0.0*	*0.7*	***0.0***	***2.0***
Median (range)	*0 (0 – 0)*	(–)	*0 (0 – 0)*	*1 (0 – 1)*	***0 (0 – 0)***	***0.5 (0 – 9)***
Total no of recorded bleeds	*0*	0	*0*	*2*	***0***	***16***
OTHER BLEEDS						
Mean	*0.1*	0	*0.3*	*1.3*	***3.0***	***1.1***
Median (range)	*0 (0 – 1)*	(–)	*0 (0 – 2)*	*1 (0 – 3)*	***3 (3 – 3)***	***1 (0 – 3)***
Total no of recorded bleeds	*1*	0	*3*	*4*	***3***	***9***

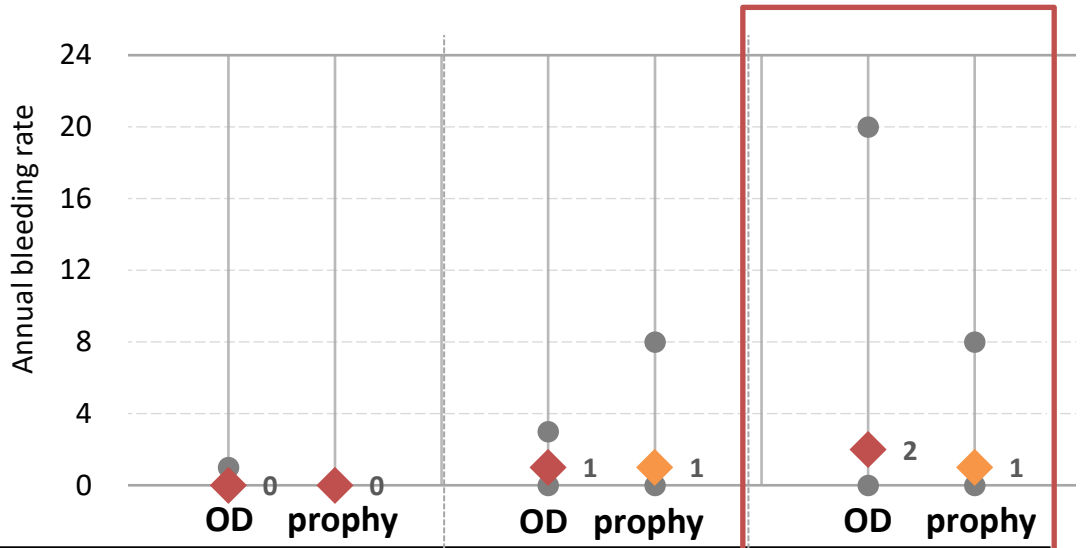
** without inhibitor*



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

Annual bleeding rate according to treatment regimen

Adults
Haem B
N=65*



Frequency of bleeding	Mild*		Moderate*		Severe*	
N total	21	0	17	3	9	15
Mean	0.2		0.9	3.0	**6.0**	**2.5**
Median (min – max)	0 (0 – 2)		1 (0 – 3)	1 (0 – 8)	**2 (0 – 20)**	**1 (0 – 11)**
Total no of recorded bleeds	4		15	9	**54**	**37**
adults on permanent prophylaxis	**0 (0%)**		**3 (15%)**		**16 (61.5%)**	
% of factor (FIX) consumed by adults on permanent prophylaxis	-		**66.6%**		**94.7%**	

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

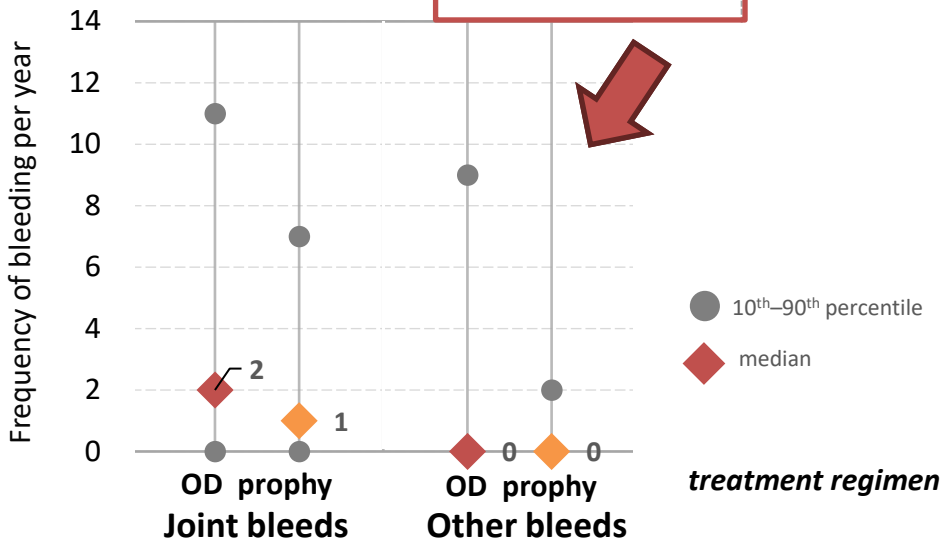
** without inhibitor;*
missing ABR in 2 adults

Joint and other bleeds according to treatment regimen

Adults
Haem B
N=65*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*21*	*0*	*17*	*3*	*9*	*15*
JOINT BLEEDS						
Mean	*0.1*	0	*0.5*	*2.0*	***4.7***	***1.9***
Median (range)	*0 (0 – 2)*	(–)	*0 (0 – 3)*	*1 (0 – 5)*	***2 (0 – 11)***	***1 (0 – 8)***
Total no of recorded bleeds	*3*	0	*9*	*6*	***42***	***29***
OTHER BLEEDS						
Mean	*0.0*	0	*0.4*	*1.0*	***1.2***	***0.5***
Median (range)	*0 (0 – 1)*	(–)	*0 (0 – 3)*	*0 (0 – 3)*	***0 (0 – 9)***	***0 (0 – 4)***
Total no of recorded bleeds	*1*	0	*6*	*3*	***11***	***8***

** without inhibitor; missing ABR in 2 adults*

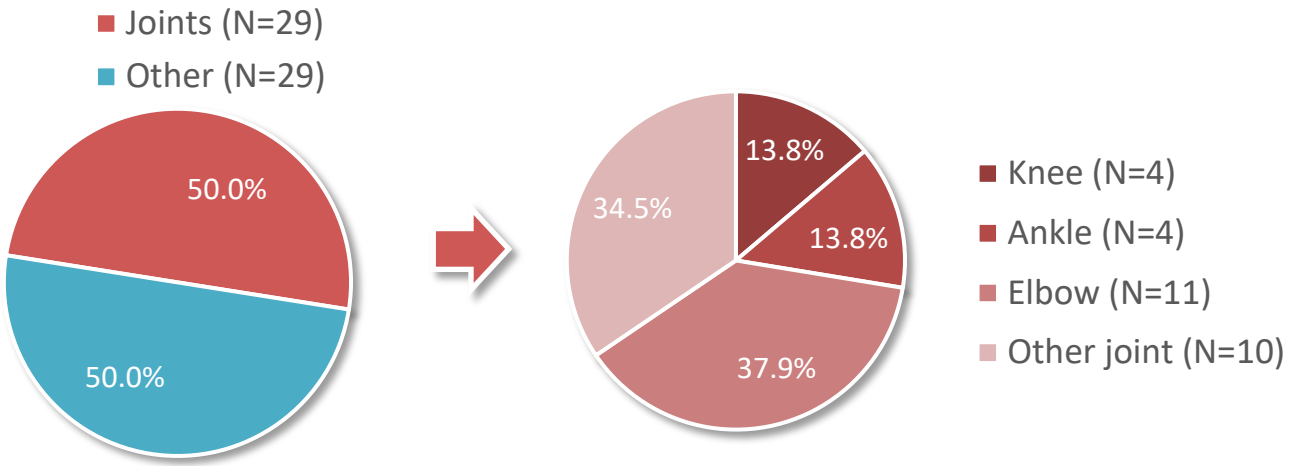


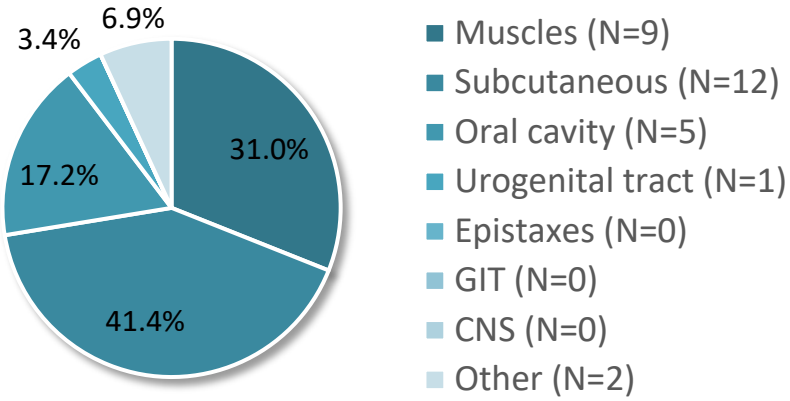
Treatment regimen:
<u>OD</u> = on demand and/or temporary prophylaxis
<u>prophy</u> = permanent prophylaxis

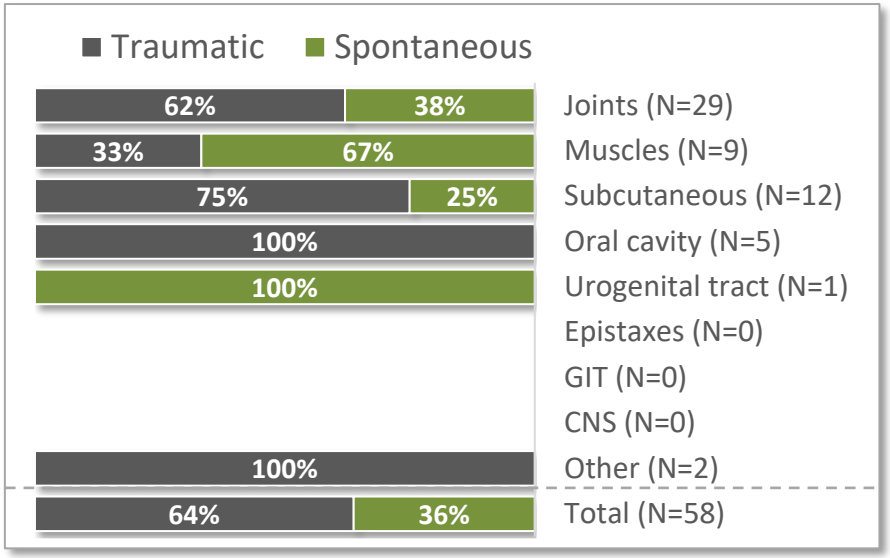
Location and etiology of bleeds

Children Haem B N=58*

** number of bleeds*







Detailed treatment of bleeds

Children Haem B N=58*

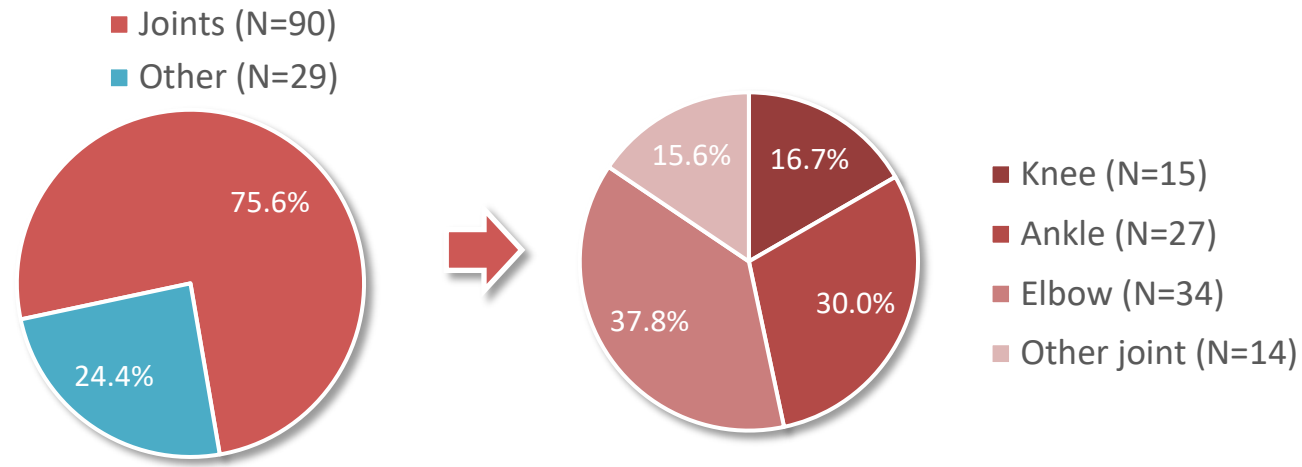
** number of bleeds*

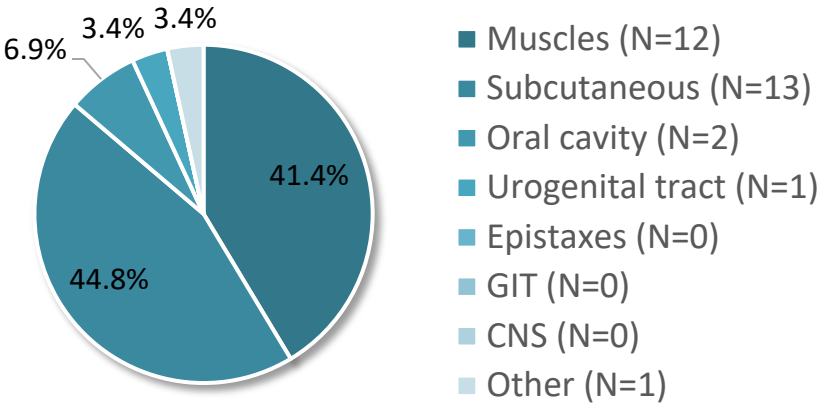
	Joints	Muscles	Subcuta-neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	*29*	*9*	*12*	*5*	*1*	*0*	*0*	*0*	*2*	***58***
FIX consumption per bleed (IU), valid N	*18*	*4*	*8*	*5*	*1*	*0*	*0*	*0*	*2*	***38***
geometric mean	2835.2	1495.3	1883.2	1653.9	2000.0				2449.5	**2227.4**
median	**2500.0**	**1500.0**	**2500.0**	**2000.0**	**2000.0**				**2500.0**	**2500.0**
min – max	1500–45000	500–8000	500–3000	500–5500	2000–2000				2000–3000	**500–45000**
sum	91000	11500	17750	13000	2000				5000	**140250**
No. of doses per bleed										
geometric mean	1.4	1.3	1.2	1.6	2.0				1.4	**1.4**
median	**1**	**1**	**1**	**1**	**2**				**2**	**1**
min – max	1–8	1–3	1–5	1–6	2–2				1–2	**1–8**
Duration of therapy per bleed, days										
geometric mean	1.5	1.6	1.4	1.5	2.0				2.0	**1.5**
median	**1**	**1**	**1**	**1**	**2**				**3**	**1**
min – max	1–27	1–6	1–10	1–7	2–2				1–4	**1–27**
N (%) with hospitalization	*0 (0%)*	*0 (0%)*	*1 (8.3%)*	*1 (20%)*	*0 (0%)*				*0 (0%)*	***2 (3.4%)***
N (%) with rebleeding	*0 (0%)*	*1 (11.1%)*	*1 (8.3%)*	*0 (0%)*	*0 (0%)*				*0 (0%)*	***2 (3.4%)***

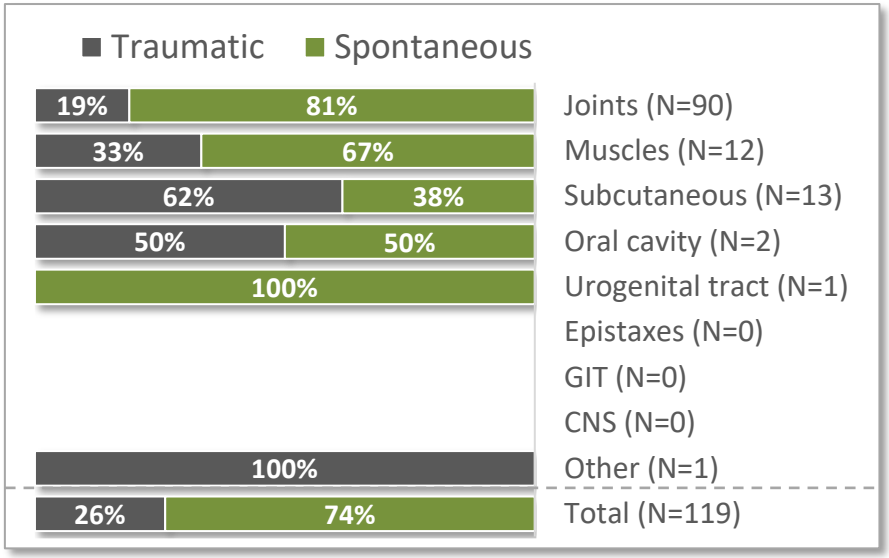
Location and etiology of bleeds

Adults
Haem B
N=119*

** number of bleeds*







Adults
Haem B
N=119*

Detailed treatment of bleeds

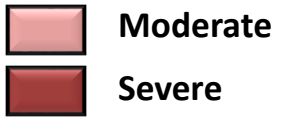
** number of bleeds*

	Joints	Muscles	Subcuta-neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	*90*	*12*	*13*	*2*	*1*	*0*	*0*	*0*	*1*	***119***
FIX consumption per bleed (IU), valid N	*90*	*12*	*13*	*2*	*1*				*1*	***119***
geometric mean	3020.3	4401.3	4844.4	2449.5	7000.0				3600.0	**3320.0**
median	**3000.0**	**4000.0**	**4000.0**	**3500.0**	**7000.0**				**3600.0**	**3600.0**
min – max	500–86400	1200–40000	1200–18000	1000–6000	7000–7000				3600–3600	**500–86400**
sum	509000	82400	85400	7000	7000				3600	**694400**
No. of doses per bleed										
geometric mean	1.8	1.5	1.7	5.5	4.0				1.0	**1.8**
median	**1**	**1**	**1**	**9**	**4**				**1**	**1**
min – max	1–20	1–15	1–6	2–15	4–4				1–1	**1–20**
Duration of therapy per bleed, days										
geometric mean	1.6	1.4	1.7	3.7	4.0				1.0	**1.6**
median	**1**	**1**	**1**	**8**	**4**				**1**	**1**
min – max	1–17	1–15	1–10	1–14	4–4				1–1	**1–17**
N (%) with hospitalization	*1 (1.1%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*				*0 (0%)*	***1 (0.8%)***
N (%) with rebleeding	*9 (10%)*	*0 (0%)*	*1 (7.7%)*	*0 (0%)*	*0 (0%)*				*0 (0%)*	***10 (8.4%)***

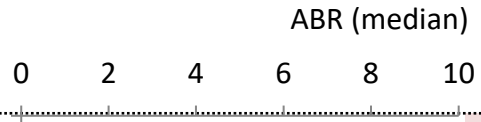
ABR according to centres Haemophilia B (PWHB)

Annual bleeding rate on permanent prophylaxis

HaemB on prophy
Paed. centres
N=11



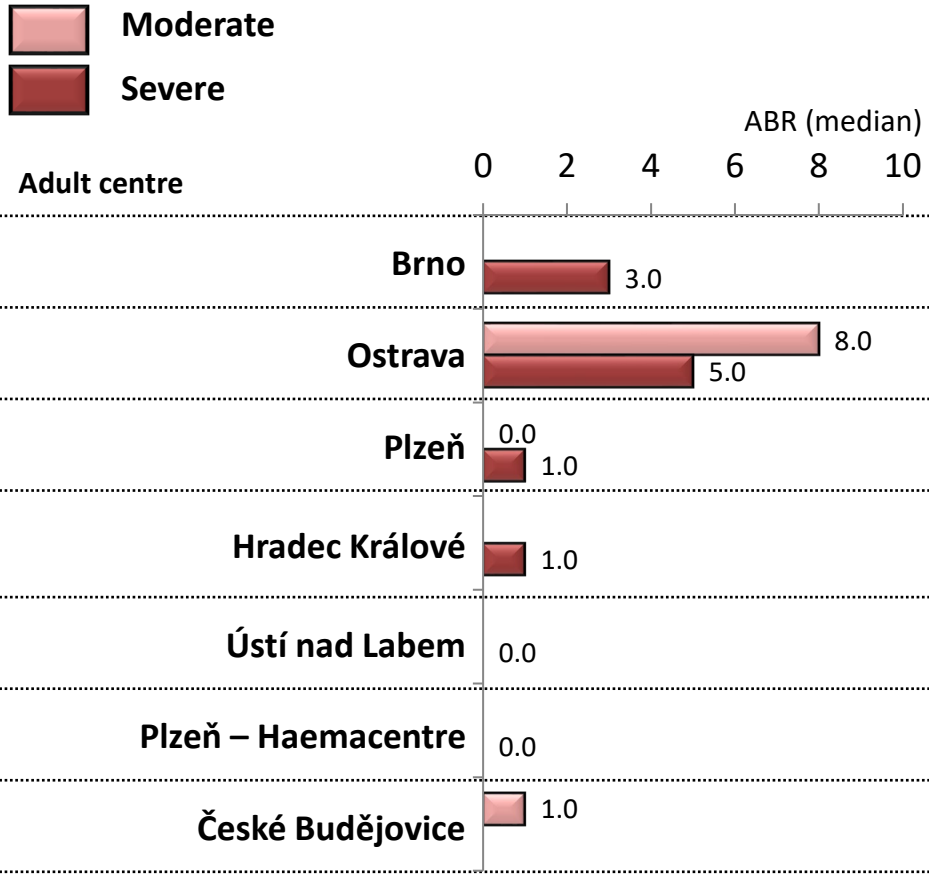
Frequency of bleeding in PWHB without inhibitor on permanent prophylaxis



Paediatric centre	N	Mean	Median	Min	Max	Severity
Praha	3	2.0	2.0	0	4	Moderate
	2	6.5	6.5	3	10	Severe
Brno	0					
	2	2.0	2.0	1	3	Severe
Ostrava	0					
	3	1.0	1.0	0	2	Severe
Ústí nad Labem	0					
	1	5.0	5.0	5	5	Severe

Annual bleeding rate on permanent prophylaxis

HaemB on prophy
Adult centres
N=18



Frequency of bleeding in PWHB without inhibitor on permanent prophylaxis

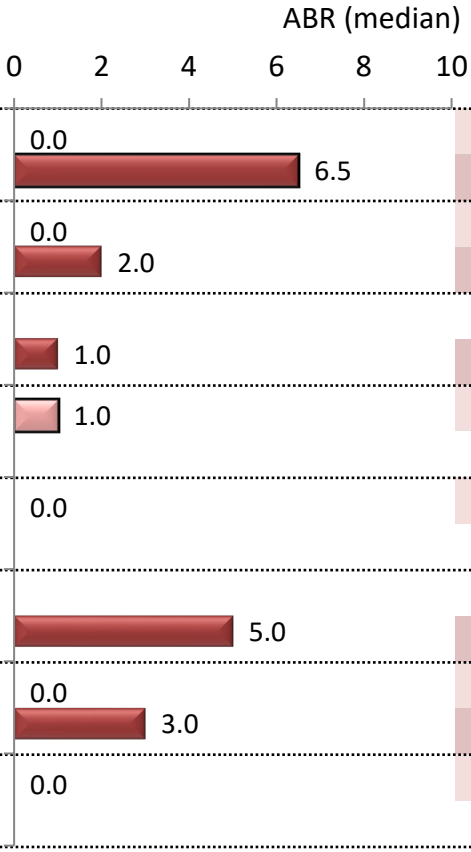
Adult centre	N	Mean	Median	Min	Max	Severity
Brno	0					
	5	3.0	3.0	0	8	Severe
Ostrava	1	8.0	8.0	8	8	Moderate
	3	5.7	5.0	1	11	Severe
Plzeň	1	0.0	0.0	0	0	Moderate
	3	1.0	1.0	0	2	Severe
Hradec Králové	0					
	2	1.0	1.0	0	2	Severe
Ústí nad Labem	0					
	1	0.0	0.0	0	0	Severe
Plzeň – Haemacentre	0					
	1	0.0	0.0	0	0	Severe
České Budějovice	1	1.0	1.0	1	1	Moderate
	0					

Annual bleeding rate regardless prophylaxis

HaemB
Paed. centres
N=23

Moderate
Severe

Frequency of bleeding in PWHB without inhibitor regardless of prophylaxis



Paediatric centre	N	Mean	Median	Min	Max	% on permanent prophylaxis
Praha	8	1.0	0.0	0	4	37.5%
	2	6.5	6.5	3	10	100.0%
Brno	2	0.0	0.0	0	0	0.0%
	2	2.0	2.0	1	3	100.0%
Ostrava	0					
	3	1.0	1.0	0	2	100.0%
České Budějovice	1	1.0	1.0	1	1	0.0%
	0					
Hradec Králové	1	0.0	0.0	0	0	0.0%
	0					
Ústí nad Labem	0					
	1	5.0	5.0	5	5	100.0%
Plzeň	1	0.0	0.0	0	0	0.0%
	1	3.0	3.0	3	3	0.0%
Olomouc	1	0.0	0.0	0	0	0.0%
	0					

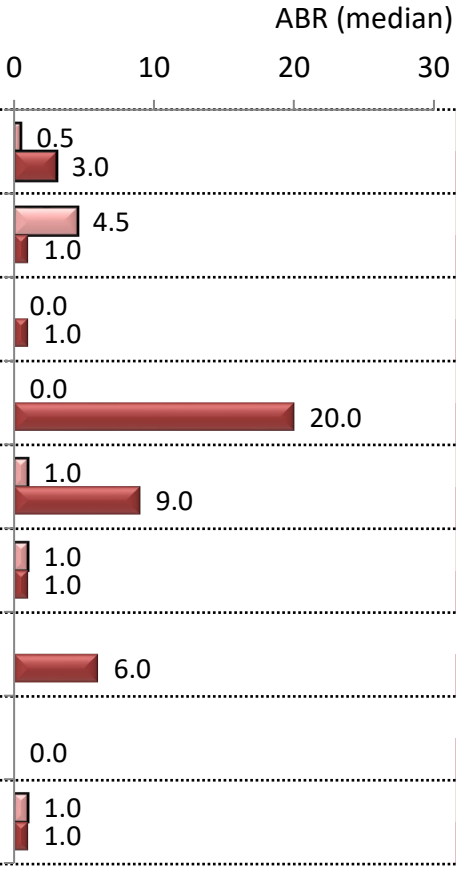
Annual bleeding rate regardless prophylaxis

HaemB
Adult centres
N=44*

** missing ABR in 2 adults*

Moderate
Severe

Frequency of bleeding in PWHB without inhibitor regardless of prophylaxis



Adult centre	N*	Mean	Median	Min	Max	% on permanent prophylaxis
Brno	4	1.0	0.5	0	3	0.0%
	5	3.0	3.0	0	8	100.0%
Ostrava	2	4.5	4.5	1	8	50.0%
	6	3.2	1.0	0	11	50.0%
Plzeň	3	0.0	0.0	0	0	33.3%
	3	1.0	1.0	0	2	100.0%
Liberec	1	0.0	0.0	0	0	0.0%
	1	20.0	20.0	20	20	0.0%
Olomouc	8	1.1	1.0	0	3	0.0%
	2	9.0	9.0	7	11	0.0%
Hradec Králové	1	1.0	1.0	1	1	0.0%
	2	1.0	1.0	0	2	100.0%
Ústí nad Labem	0					
	2	6.0	6.0	0	12	50.0%
Plzeň – Haemacentre	0					
	1	0.0	0.0	0	0	100.0%
České Budějovice	1	1.0	1.0	1	1	100.0%
	2	1.0	1.0	0	2	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	8	**37.5%**	3	65.5	**65.4**	62.3	68.7	0.0	**0.0**	5	5.0	16.0
	Severe	2	**100.0%**	2	35.7	**35.7**	31.1	40.4	0.0	**0.0**	0		
Brno	Moderate	2	**0.0%**	0							2	2.0	6.0
	Severe	2	**100.0%**	2	35.3	**35.3**	34.0	36.5	0.0	**0.0**	0		
Ostrava	Moderate	0	**0.0%**	0							0		
	Severe	3	**100.0%**	3	45.9	**41.7**	28.0	68.2	0.0	**0.0**	0		
Č. Budějovice	Moderate	1	**0.0%**	0							1	1.0	3.0
	Severe	0	**0.0%**	0							0		
Hradec Králové	Moderate	1	**0.0%**	0							1	1.0	4.0
	Severe	0	**0.0%**	0							0		
Ústí nad Labem	Moderate	0	**0.0%**	0							0		
	Severe	1	**100.0%**	1	69.9	**69.9**	69.9	69.9	0.0	**0.0**	0		
Plzeň	Moderate	1	**0.0%**	0							1	1.0	2.0
	Severe	1	**0.0%**	0							1	1.0	6.0
Olomouc	Moderate	1	**0.0%**	0							1	1.0	3.0
	Severe	0	**0.0%**	0							0		

Prophylactic regimens and treatment outcomes

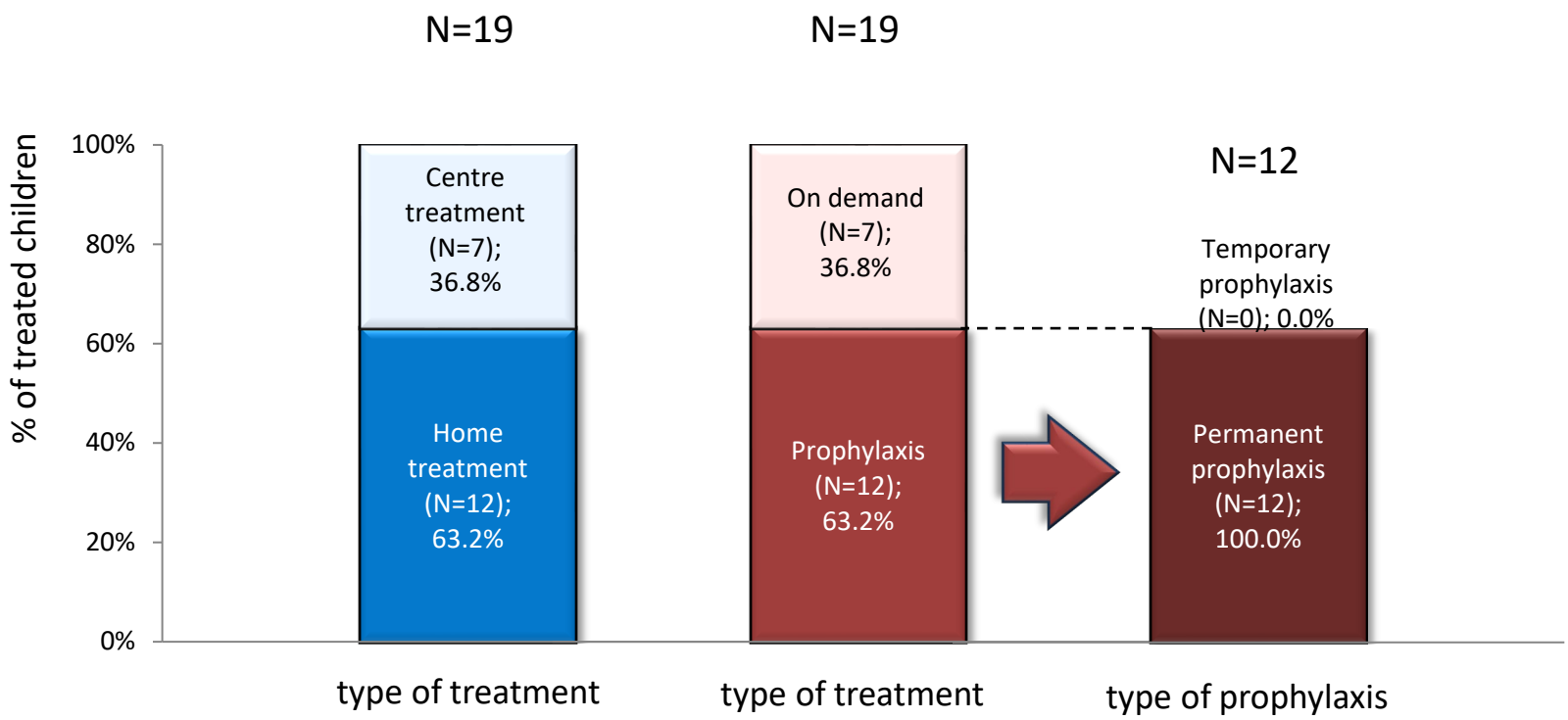
HaemB
Adult centres
N=46

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	1.0	0.5	50
	Severe	5	**100.0%**	5	64.1	**54.5**	46.3	101.4	3.0	**3.0**	32	0			
Ostrava	Moderate	2	**50.0%**	1	50.0	**50.0**	50.0	50.0	8.0	**8.0**	20	1	1.0	1.0	36
	Severe	6	**50.0%**	3	54.1	**52.2**	47.6	62.5	5.7	**5.0**	52	3	0.7	1.0	60
Plzeň	Moderate	3	**33.3%**	1	15.3	**15.3**	15.3	15.3	0.0	**0.0**	36	2	0.0	0.0	63
	Severe	4	**100.0%**	4	21.3	**19.0**	15.7	31.6	1.0	**1.0**	40	0			
Liberec	Moderate	1	**0.0%**	0								1	0.0	0.0	46
	Severe	1	**0.0%**	0								1	20.0	20.0	27
Olomouc	Moderate	8	**0.0%**	0								8	1.1	1.0	49
	Severe	3	**0.0%**	0								3	9.0	9.0	50
Hradec Králové	Moderate	1	**0.0%**	0								1	1.0	1.0	65
	Severe	2	**100.0%**	2	29.2	**29.2**	20.0	38.4	1.0	**1.0**	41	0			
Ústí n. Labem	Moderate	0	**0.0%**	0								0			
	Severe	2	**50.0%**	1	53.0	**53.0**	53.0	53.0	0.0	**0.0**	24	1	12.0	12.0	48
Plzeň - Haemacentre	Moderate	0	**0.0%**	0								0			
	Severe	1	**100.0%**	1	44.6	**44.6**	44.6	44.6	0.0	**0.0**	38	0			
Č. Budějovice	Moderate	1	**100.0%**	1	26.7	**26.7**	26.7	26.7	1.0	**1.0**	53	0			
	Severe	2	**0.0%**	0								2	1.0	1.0	51

** missing ABR in 2 adults*

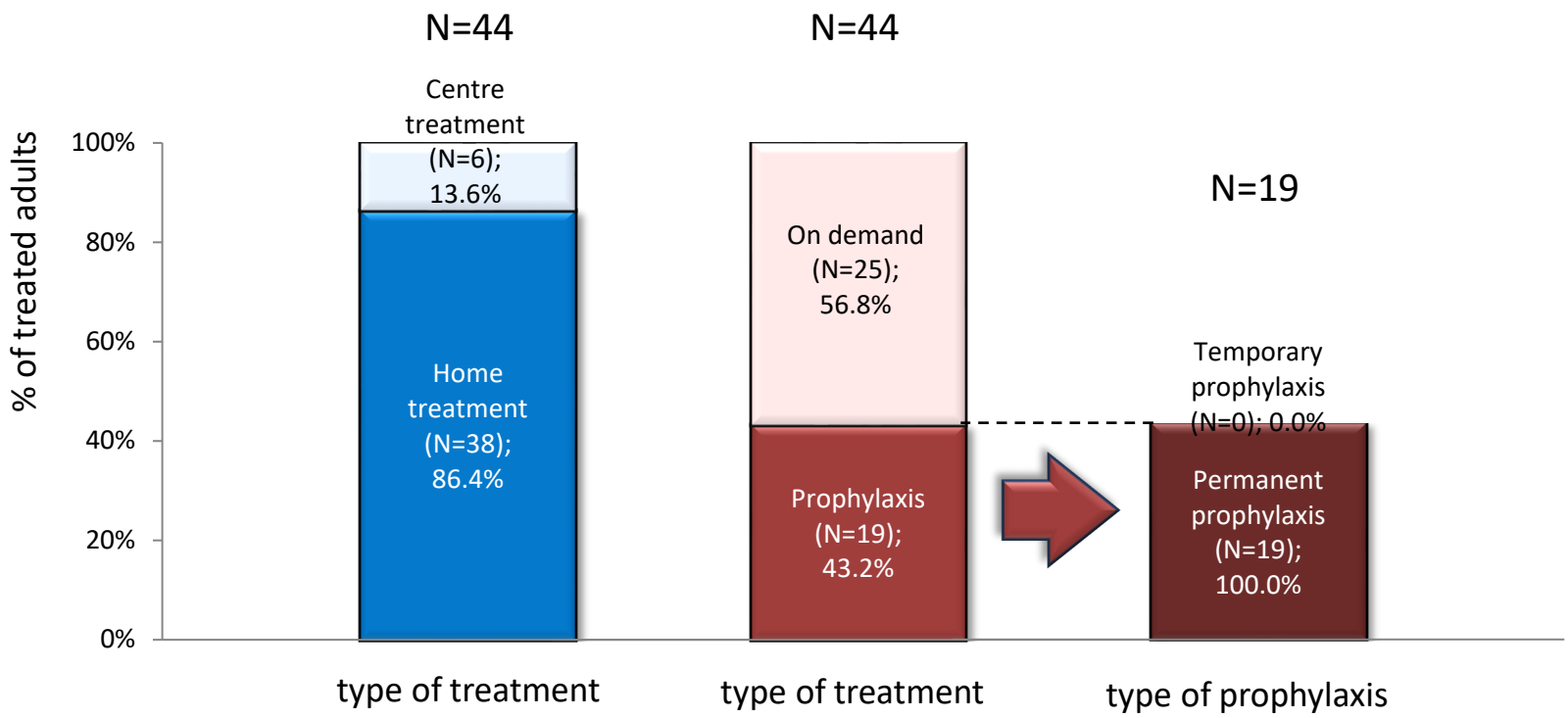
Children
Haem B
N=19

Type of treatment (subgroup of treated patients)



Adults
Haem B
N=44

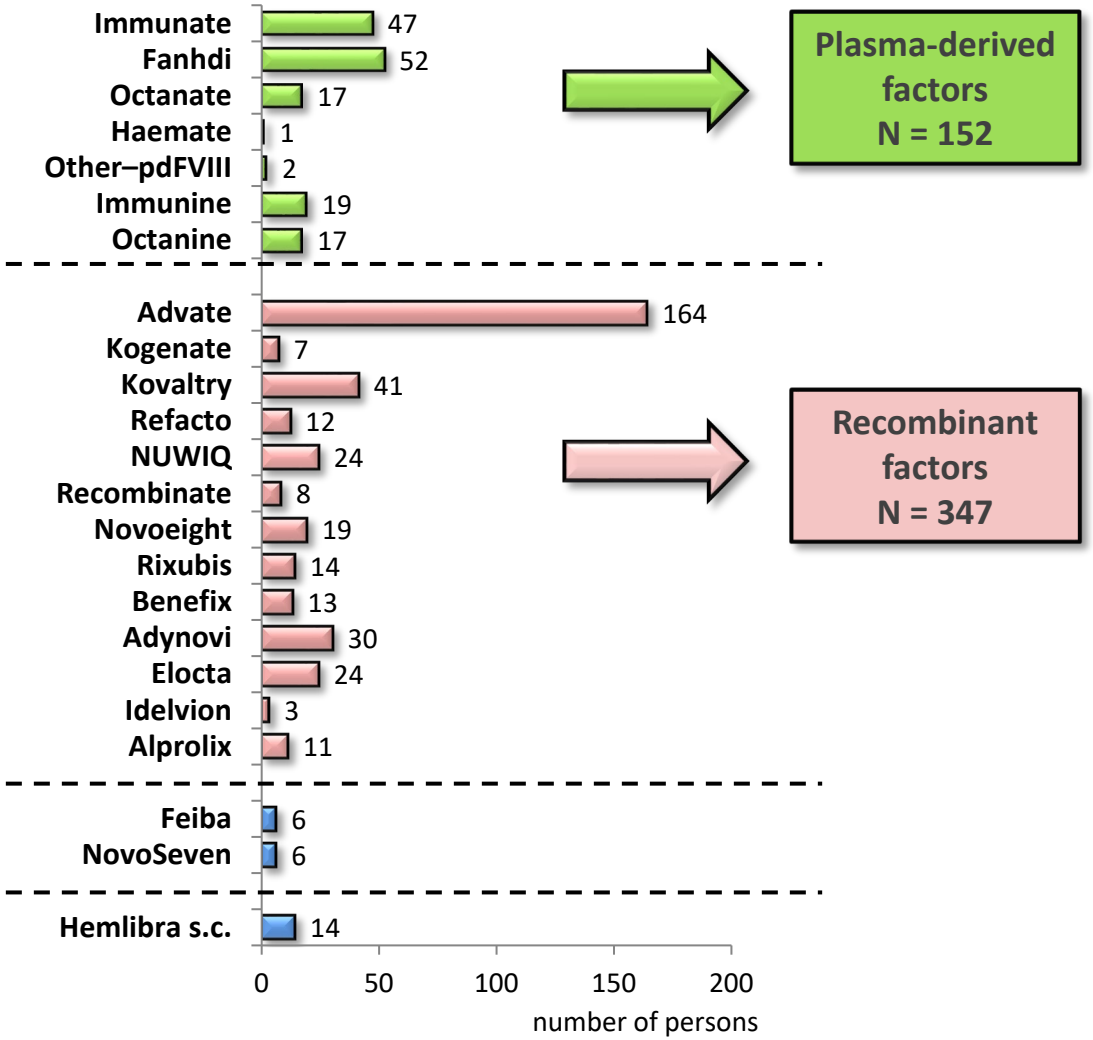
Type of treatment (subgroup of treated patients)



Treatment data and factor consumption Haemophilia A and B

Treatment

All
N=421[1]

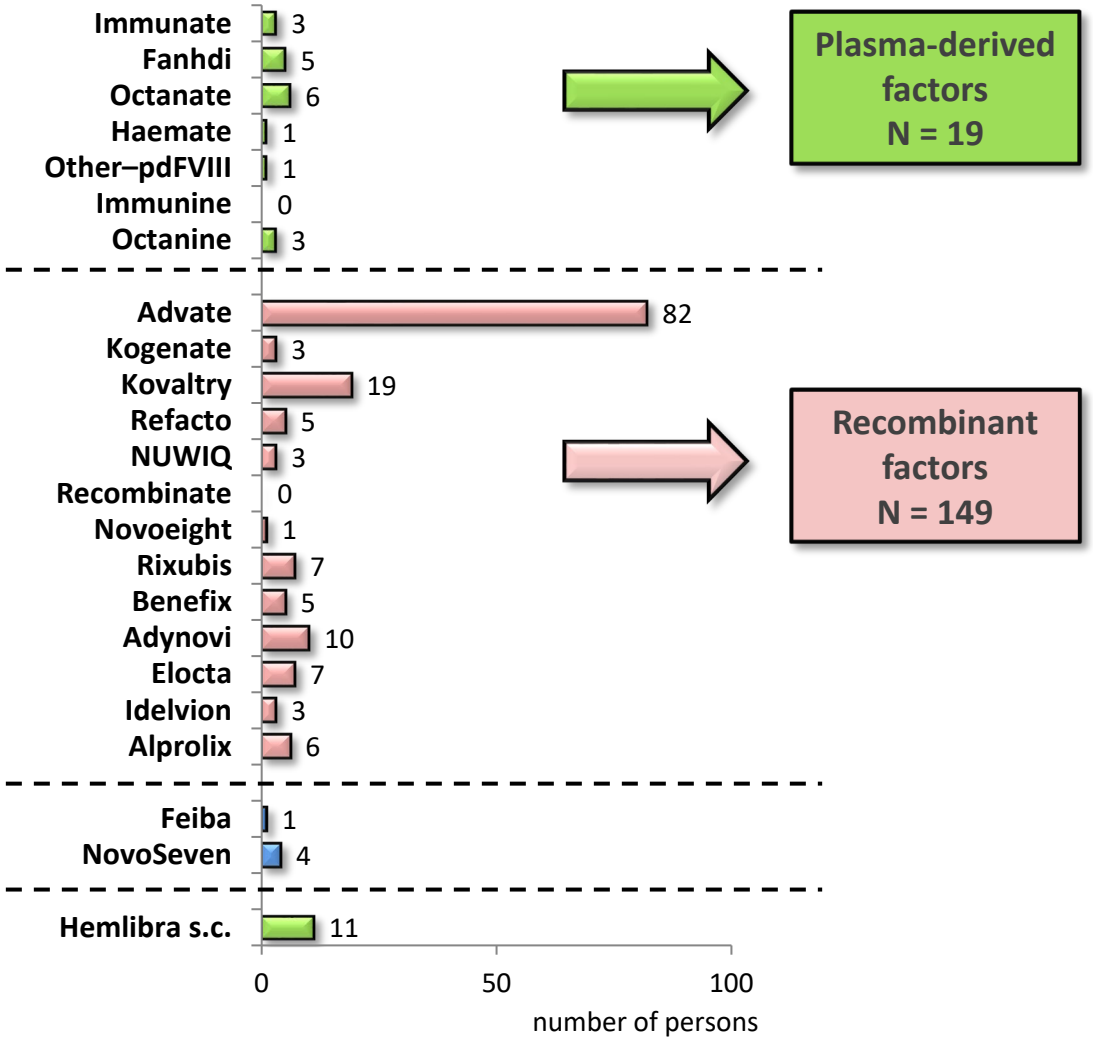


423 persons (52.9% of all PWH) were treated in 2019 (**409 persons received standard factor concentrates**, 78 persons received EHL factors, by-pass therapy or emicizumab, in 2 data are not available; 96 persons received more than one type/brand of concentrate).
22 persons were treated with both plasma-derived and recombinant factor.

[1]missing type of treatment in 1 adult and 1 child

Treatment

Children
N=147[1]

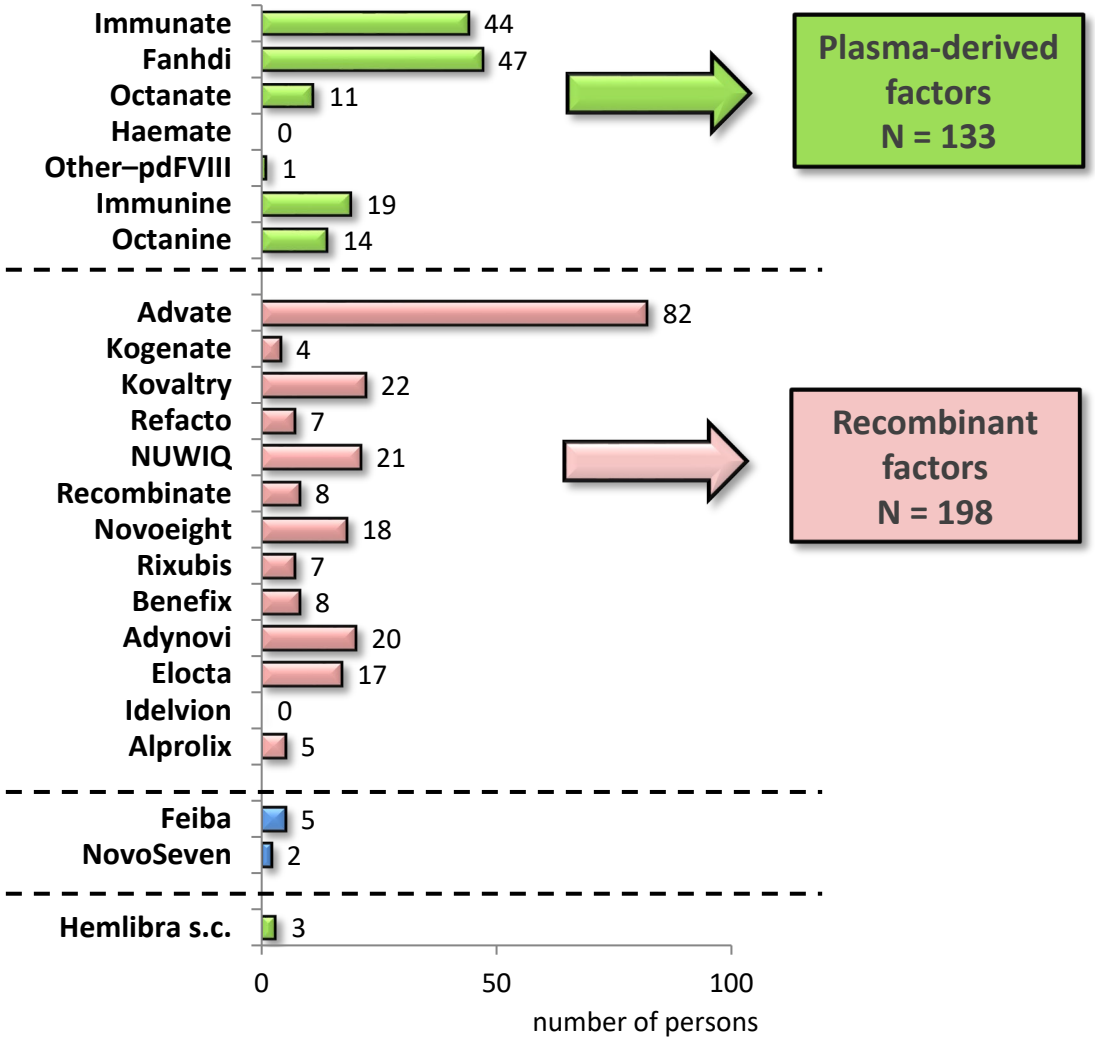


148 children (56.1% of all PWH) were treated in 2019 (**140 children received standard factor concentrates**, 31 children EHL factors, by-pass therapy or emicizumab, in 1 data are not available; 33 persons received more than one type/brand of concentrate).
Two children were treated with both plasma-derived and recombinant factor.

[1]*missing type of treatment in 1 child*

Treatment

Adults
N=274[1]



275 adults (51.3% of all PWH) were treated in 2019 (**269 adults received standard factor concentrates**, 47 adults EHL factors, by-pass therapy or emicizumab, in 1 data are not available; 63 adults received more than one type/brand of concentrate).
20 adults were treated with both plasma-derived and recombinant factor.

[1]missing type of treatment in 1 adult

All

Comparison of treatment in years 2019 and 2018

	2019			2018		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	412	51.5	100.0	404	52.5	100.0
Plasma-derived factor	*152*	*19.0*	*36.9*	*182*	*23.6*	*45.0*
Recombinant factor	*279*	*34.9*	*67.7*	*247*	*32.1*	*61.1*
Recombinant f. EHL	*68*	*8.5*	*16.5*	*3*	*0.4*	*0.7*
Without treatment	388	48.5	-	366	47.5	-
Total	800	100.0	-	770	100.0	-

** One patient could have more type of factor concentrates.*

Children

Comparison of treatment in years 2019 and 2018

	2019			2018		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	143	54.2	100.0	133	53.4	100.0
Plasma-derived factor	*19*	*7.2*	*13.3*	*27*	*10.8*	*20.3*
Recombinant factor	*123*	*46.6*	*86.0*	*109*	*43.8*	*82.0*
Recombinant f. EHL	*26*	*9.8*	*18.2*	*3*	*1.2*	*2.3*
Without treatment	121	45.8	-	116	46.6	-
Total	264	100.0	-	249	100.0	-

** One patient could have more type of factor concentrates.*

Adults

Comparison of treatment in years 2019 and 2018

	2019			2018		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	269	50.2	100.0	271	52.0	100.0
Plasma-derived factor	*133*	*24.8*	*49.4*	*155*	*29.8*	*57.2*
Recombinant factor	*156*	*29.1*	*58.0*	*138*	*26.5*	*50.9*
Recombinant f. EHL	*42*	*7.8*	*15.6*	*0*	*0.0*	*0.0*
Without treatment	267	49.8	-	250	48.0	-
Total	536	100.0	-	521	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)	Immunate	3 478 500	47	74 010.6
	Fanhdi	4 176 450	52	80 316.3
	Octanate	1 798 000	17	105 764.7
	Haemate P	7 000	1	7 000.0
	Other plasma-derived	294 000	2	147 000.0
	FVIII PD total	**9 753 950**	**116**	**84 085.8**
	Advate	17 953 750	164	109 474.1
	Kogenate	282 000	7	40 285.7
	Kovaltry	4 201 000	41	102 463.4
	Refacto	1 509 000	12	125 750.0
	NUWIQ	2 626 250	24	109 427.1
	Recombinate	257 500	8	32 187.5
	Novoeight	3 203 500	19	168 605.3
	FVIII REC total	**30 033 000**	**252**	**119 178.6**
	Standard FVIII total	39 786 950	296	134 415.4
	Adynovi	3 828 400	30	127 613.3
	Elocta	2 402 000	24	100 083.3
	FVIII REC EHL total	6 230 400	54	115 377.8
	FVIII total	**46 017 350**	**349**	**131 854.9**
FIX (IU)	Immunine	759 000	19	39 947.4
	Octanine	894 500	17	52 617.6
	FIX PD total	**1 653 500**	**36**	**45 930.6**
	Rixubis	2 299 250	14	164 232.1
	Benefix	1 407 250	13	108 250.0
	FIX REC total	**3 706 500**	**27**	**137 277.8**
	Standard FIX total	38 315 600	349	109 786.8
	Idelvion	486 500	3	162 166.7
	Alprolix	484 500	11	44 045.5
	FIX REC EHL total	971 000	14	69 357.1
	FIX total	**5 113 700**	**61**	**83 831.1**
By-pass	Feiba (U)	1 508 000	6	251 333.3
	NovoSeven (mg)	2 164.4	6	360.7
Emicizumab	Hemlibra s.c. (mg)	29 643	14	2 117.4

Consumption of drugs

Children

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	Immunate	168 000	3	56 000.0
	Fanhdi	456 450	5	91 290.0
	Octanate	846 000	6	141 000.0
	Haemate P	7 000	1	7 000.0
	Other plasma-derived	284 000	1	284 000.0
	FVIII PD total	***1 761 450***	***16***	***110 090.6***
	Advate	7 484 750	82	91 277.4
	Kogenate	77 500	3	25 833.3
	Kovaltry	1 813 000	19	95 421.1
	Refacto	557 000	5	111 400.0
	NUWIQ	424 250	3	141 416.7
	Recombinate	0	0	
	Novoeight	650 000	1	650 000.0
	FVIII REC total	***11 006 500***	***111***	***99 157.7***
	Standard FVIII total	**12 767 950**	**107**	**119 326.6**
	Adynovi	777 500	10	77 750.0
	Elocta	407 500	7	58 214.3
	FVIII REC EHL total	***1 185 000***	***17***	***69 705.9***
	FVIII total	**13 952 950**	**123**	**113 438.6**
FIX (IU)	Immunine	0	0	
	Octanine	25 500	3	8 500.0
	FIX PD total	***25 500***	***3***	***8 500.0***
	Rixubis	593 750	7	84 821.4
	Benefix	196 250	5	39 250.0
	FIX REC total	***790 000***	***12***	***65 833.3***
	Standard FIX total	**815 500**	**9**	**90 611.1**
	Idelvion	486 500	3	162 166.7
	Alprolix	196 000	6	32 666.7
	FIX REC EHL total	***682 500***	***9***	***75 833.3***
	FIX total	**1 498 000**	**18**	**83 222.2**
By-pass	Feiba (U)	189 000	1	189 000.0
	NovoSeven (mg)	1 395.4	4	348.9
Emicizumab	Hemlibra s.c. (mg)	18 003	11	1 636.6

Consumption of drugs

Adults

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	Immunate	3 310 500	44	75 238.6
	Fanhdi	3 720 000	47	79 148.9
	Octanate	952 000	11	86 545.5
	Haemate P	0	0	
	Other plasma-derived	10 000	1	10 000.0
	FVIII PD total	***7 992 500***	***100***	***79 925.0***
	Advate	10 469 000	82	127 670.7
	Kogenate	204 500	4	51 125.0
	Kovaltry	2 388 000	22	108 545.5
	Refacto	952 000	7	136 000.0
	NUWIQ	2 202 000	21	104 857.1
	Recombinate	257 500	8	32 187.5
	Novoeight	2 553 500	18	141 861.1
	FVIII REC total	***19 026 500***	***141***	***134 939.7***
	Standard FVIII total	**27 019 000**	**189**	**142 957.7**
	Adynovi	3 050 900	20	152 545.0
	Elocta	1 994 500	17	117 323.5
	FVIII REC EHL total	***5 045 400***	***37***	***136 362.2***
	FVIII total	**32 064 400**	**226**	**141 877.9**
FIX (IU)	Immunine	759 000	19	39 947.4
	Octanine	869 000	14	62 071.4
	FIX PD total	***1 628 000***	***33***	***49 333.3***
	Rixubis	1 705 500	7	243 642.9
	Benefix	1 211 000	8	151 375.0
	FIX REC total	***2 916 500***	***15***	***194 433.3***
	Standard FIX total	**4 544 500**	**38**	**119 592.1**
	Idelvion	0	0	
	Alprolix	288 500	5	57 700.0
	FIX REC EHL total	***288 500***	***5***	***57 700.0***
	FIX total	**4 833 000**	**43**	**112 395.3**
By-pass	Feiba (U)	1 319 000	5	263 800.0
	NovoSeven (mg)	769.0	2	384.5
Emicizumab	Hemlibra s.c. (mg)	11 640	3	3 880.0