The status of care for persons with haemophilia registered within CNHP registry
Annual Report 2023

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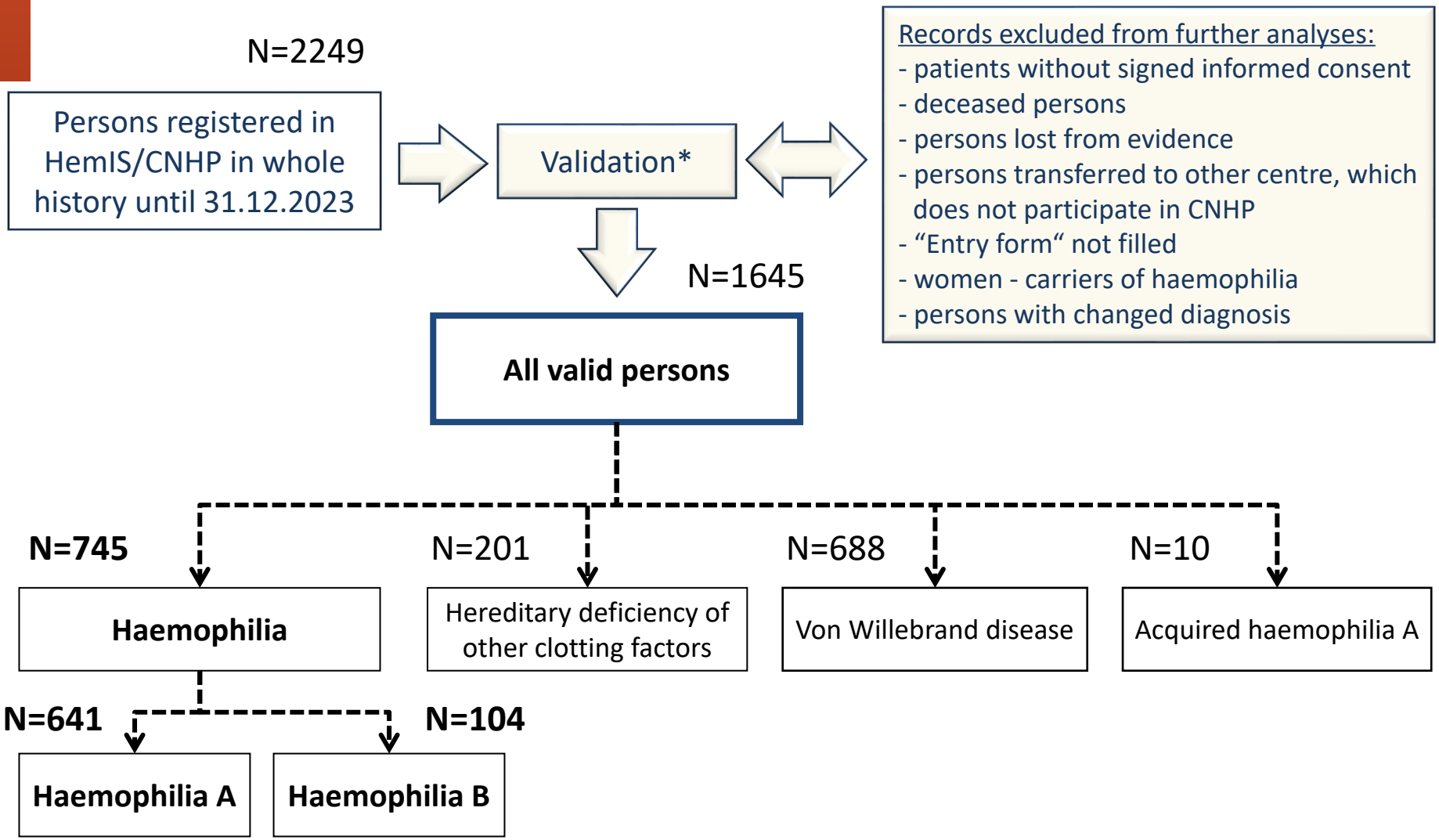
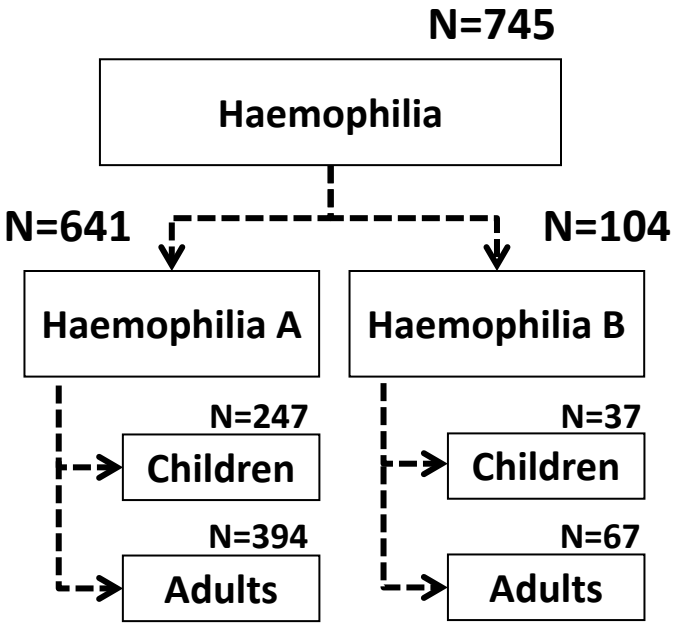
on behalf of
Centres contributing to CNHP registry
(Czech National Haemophilia Programme)

Export date: April 2, 2024

Sample size, valid records



Persons with haemophilia (PWH)



Centres participating in CNHP

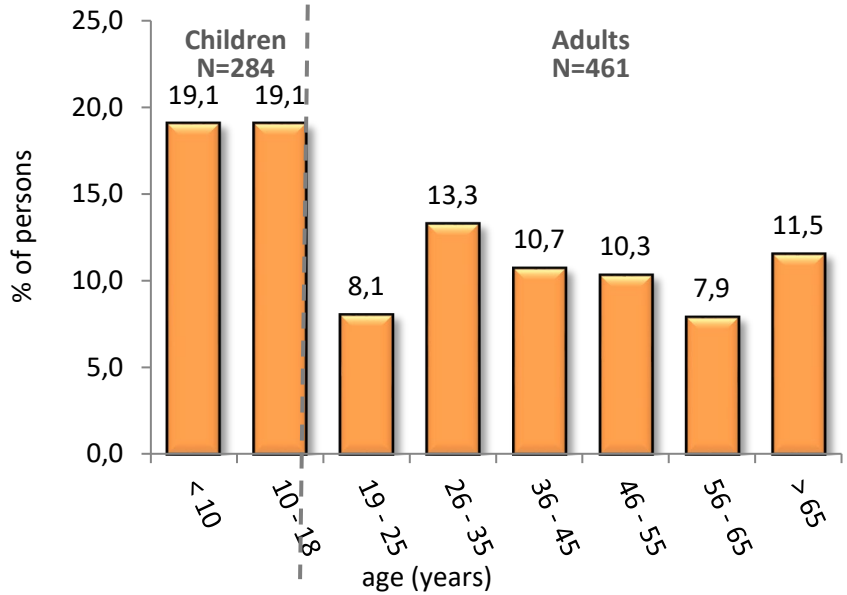
Paediatric centres	Valid persons N	%
Prague – Dpt. of Pediatric Haematology and Oncology, CUH Motol	106	14.2
Brno – Dpt. of Pediatric Haematology, CUH Brno	70	9.4
Hradec Králové – Dpt. of Pediatric Medicine, UH HK	30	4.0
Ostrava – Dpt. of Pediatric Medicine, UH Ostrava	25	3.4
Ústí n.L. – Pediatric Dpt. – Haematology, Masaryk Hospital	18	2.4
Olomouc – Dpt. of Pediatric Medicine, UH Olomouc	15	2.0
České Budejovice – Pediatric Dpt., Hospital CB	15	2.0
Pilsen – Pediatric Dpt., UH Pilsen	12	1.6

Adult centres	Valid persons N	%
Brno – Dpt. Of Clin Hematol, UH Brno	174	23.4
Ostrava – Blood centre, UH Ostrava	68	9.1
Olomouc – Haemato-Oncology Dpt., UH Olomouc	57	7.7
Pilsen – Dpt. of Biochemistry and Hematology, UH Pilsen	53	7.1
Liberec – Dpt. Of Clin Hematol, Hospital Liberec	47	6.3
Ústí n.L. – Dpt. Of Clin Hematol, Masaryk Hospital	29	3.9
České Budějovice – Dpt. Of Clin Hematol, Hospital CB	26	3.5

Basic demographics

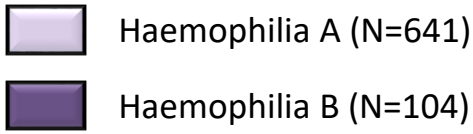
All
N=745

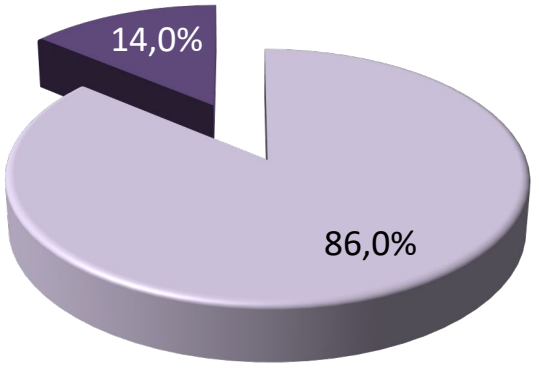
	Actual age* (years)
N	*745*
Mean	*32.2*
Median (min - max)	*29 (0 – 88)*



** age reached in year 2023*

Type of haemophilia





One child with haemophilia was born in 2023.

All
N=745

Persons with haemophilia and inhibitors in 2023

Active inhibitors were recorded in <u>18 persons</u> during 2023

- 2 inhibitors in children with severe HA newly developed and one adult had re-occurence in 2023

PWH with inhibitors:

- 11 children and 7 adults
- 17 haemophilia A and 1 haemophilia B
- 16 in severe and 2 in moderate haemophilia
- 12 high-titre and 6 low-titre (<5BU)
- 10 high response and 6 low response inhibitors; this information not available in 2 PWH with inhibitor
- 16 patients were treated with emicizumab
  - 10 patients were treated only with emi, 4 patients with emi and by-pass therapy, and 2 patients with emi and FVIII during the year
- 1 patient with haemophilia B was treated with rFVIIa and Feiba
- One patient has already been on-going ITT in 2023 (started earlier)

Eradication of inhibitor:

- Seven patients with a history of inhibitor (now receiving emicizumab prophylaxis) had repeatedly negative inhibitor levels and were considered inhibitor-free this year
- One patient with inhibitor died

ABR and treatment regimens in patients with inhibitor

N=18

	Type	Year of birth	Severity	ITT	Emi	By-pass	Titre	Responder	ABR	Joint / other
1	HA	2022	**Severe**		**Yes**		low	LR	**1**	0 / 1
2	HA	2021	**Severe**		**Yes**		low	LR	0	0 / 0
3	HA	2021	**Severe**		**Yes**		low	LR	0	0 / 0
4	HA	2021	**Severe**		**Yes**		low	NA	0	0 / 0
5	HA	2021	**Severe**		**Yes**	OD	**high**	**HR**	**2**	0 / 2
6	HA	2020	**Severe**	Yes	**Yes**		**high**	NA	0	0 / 0
7	HA	2018	**Severe**		**Yes**		low	LR	0	0 / 0
8	HA	2017	**Severe**		**Yes**		**high**	**HR**	0	0 / 0
9	HA	2015	**Severe**		**Yes**	OD	**high**	**HR**	**1**	0 / 1
10	HA	2004	**Severe**		**Yes**		**high**	**HR**	0	0 / 0
11	HA	1977	**Severe**		**Yes**	OD	**high**	**HR**	0	0 / 0
12	HA	1976	**Severe**				low	LR	0	0 / 0
13	HA	1975	**Severe**		**Yes**		**high**	**HR**	0	0 / 0
14	HA	1971	**Severe**		**Yes**		**high**	**HR**	0	0 / 0
15	HA	1956	**Severe**		**Yes**		**high**	LR	0	0 / 0
16	HA	2011	Moderate		**Yes**	OD	**high**	**HR**	**1**	0 / 1
17	HA	1941	Moderate		**Yes**		**high**	**HR**	0	0 / 0
18	HB	2007	Severe			Permanent px	**high**	**HR**	**16**	8 / 8

new in 2023

NA not available

N=18

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 px	1	0.00	0 (0-0)	0 / 0
		OD	0	-	-	-
	No	Emi px	15	0.33	0 (0-2)	0 / 0
		OD	1	0.00	0 (0-0)	0 / 0
Haemophilia B	No	BPA permanent	1	16.00	16 (16-16)	8 / 8

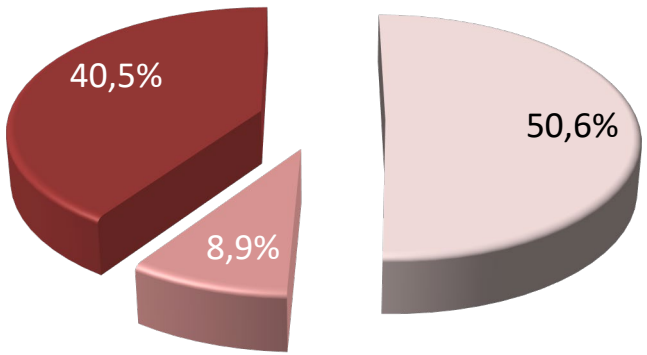
Demographic characteristics Haemophilia A

Severity of haemophilia A

Haem A
N=641

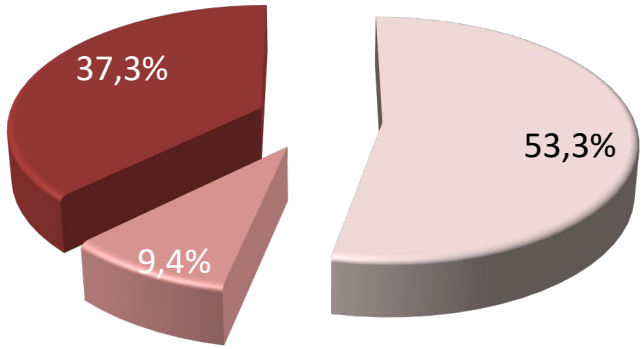
Children (N=247)

- Mild (N=125)
- Moderate (N=22)
- Severe (N=100)



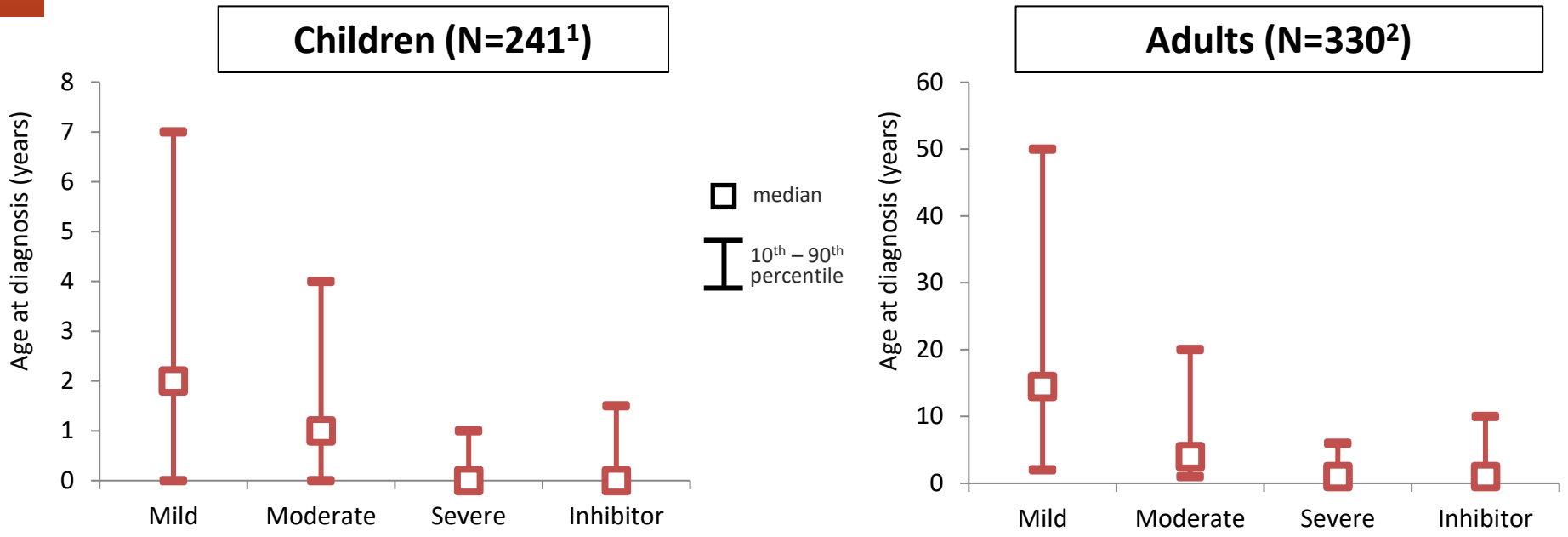
Adults (N=394)

- Mild (N=210)
- Moderate (N=37)
- Severe (N=147)



Haem A
N=641

Age at diagnosis according to severity of haemophilia A



Mild*	Moderate*	Severe*	Inhibitor+	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor+
123	*22*	*96*	*10*	**N valid**	*186*	*32*	*112*	*7*
2.7	*1.7*	*0.6*	*0.4*	**Mean**	*20.5*	*7.7*	*2.3*	*2.4*
2 (0 – 16)	*1 (0 – 10)*	*0 (0 – 7)*	*0 (0 – 2)*	**Median (min – max)**	*14.5 (0 – 68)*	*4 (0 – 32)*	*1 (0 – 38)*	*1 (0 – 10)*

[1] *Missing information on year of diagnosis in 6 children.*

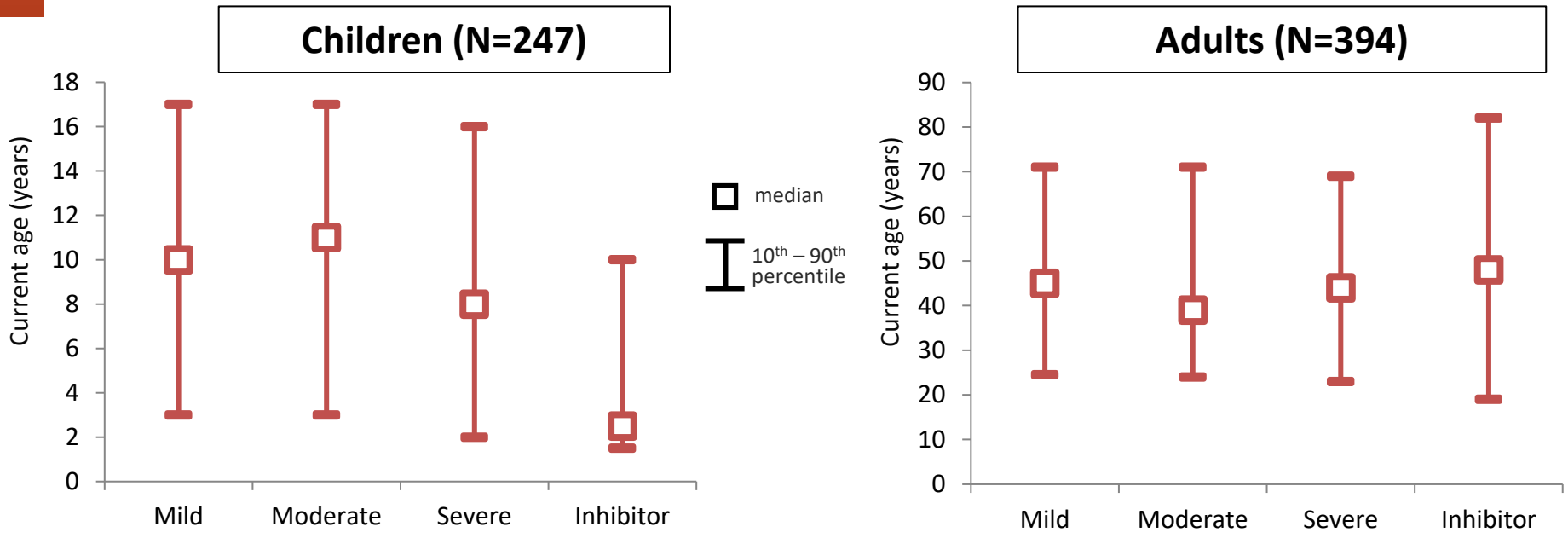
[2] *Missing information on year of diagnosis in 64 adults.*

** including persons with inhibitor*

+ in 2023

Haem A
N=641

Actual age according to severity of haemophilia A



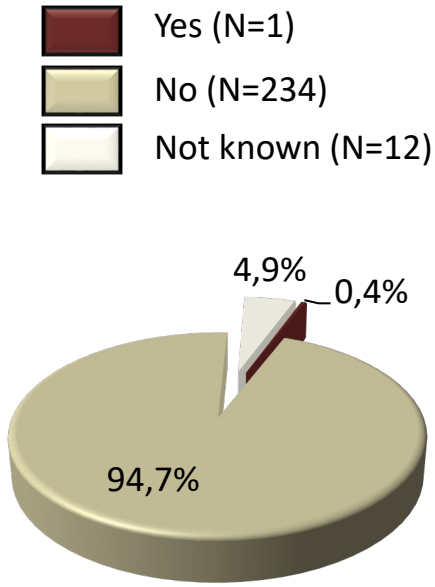
Mild*	Moderate*	Severe*	Inhibitor+	Current age++ (years)	Mild*	Moderate*	Severe*	Inhibitor+
125	*22*	*100*	*10*	**N valid**	*210*	*37*	*147*	*7*
10.2	*10.8*	*8.6*	*4.3*	**Mean**	*46.7*	*44.4*	*44.4*	*51.6*
10 (0 – 18)	*11 (2 – 18)*	*8 (1 – 18)*	*2.5 (1 – 12)*	**Median (min – max)**	*45 (19 – 88)*	*39 (20 – 82)*	*44 (19 – 84)*	*48 (19 – 82)*

** including persons with inhibitor*
+ in 2023
++ age reached in year 2023

Hepatitis (ever) experienced

Children
Haem A
N=247

Experienced hepatitis



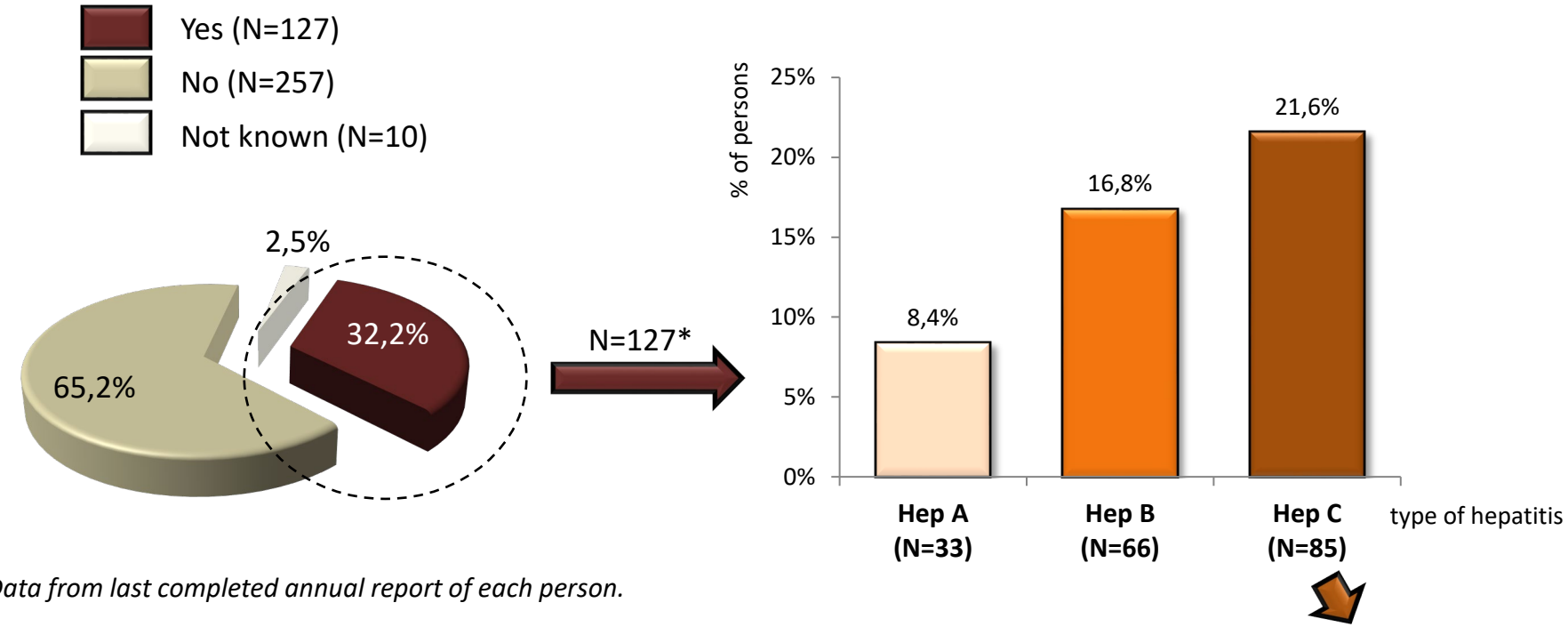
One child has hepatitis A.

Data from last completed annual report of each person.

Hepatitis (ever) experienced

Adults
Haem A
N=394

Experienced hepatitis



Data from last completed annual report of each person.

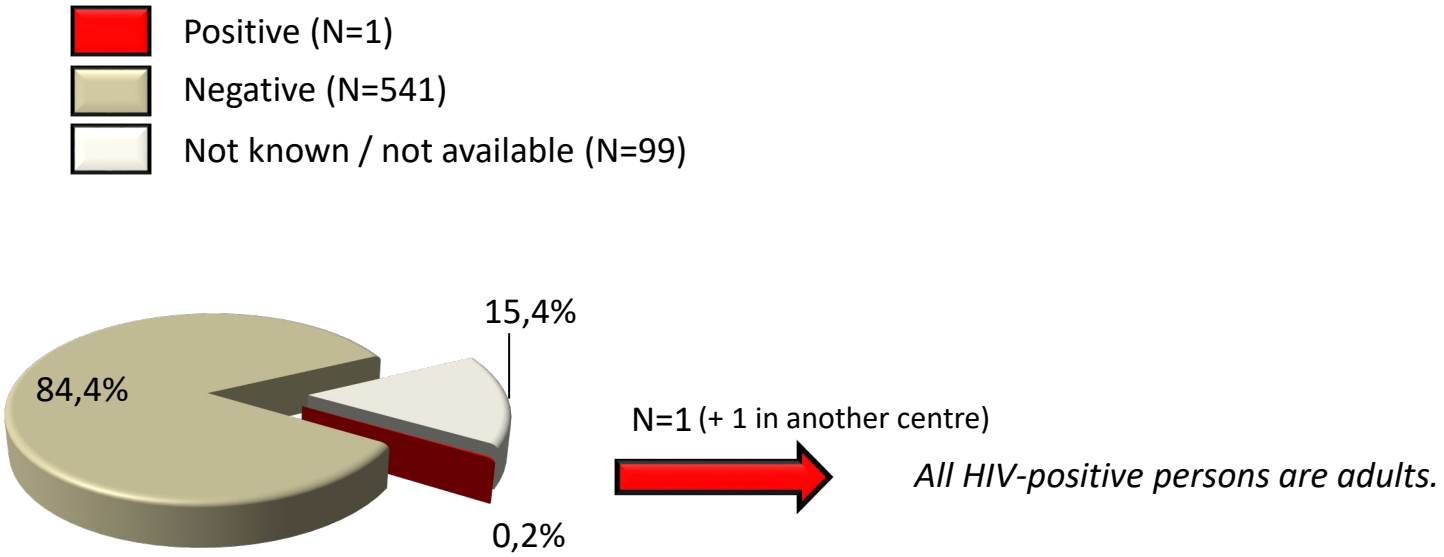
**Total of 184 cases of hepatitis in 127 persons. One person may have more types of hepatitis recorded.*

14 adults are HCV RNA positive

HIV

All
Haem A
N=641

HIV



All HIV-positive persons are adults.

Data from last completed annual report of each person.

Treatment outcomes and bleeding frequency Haemophilia A

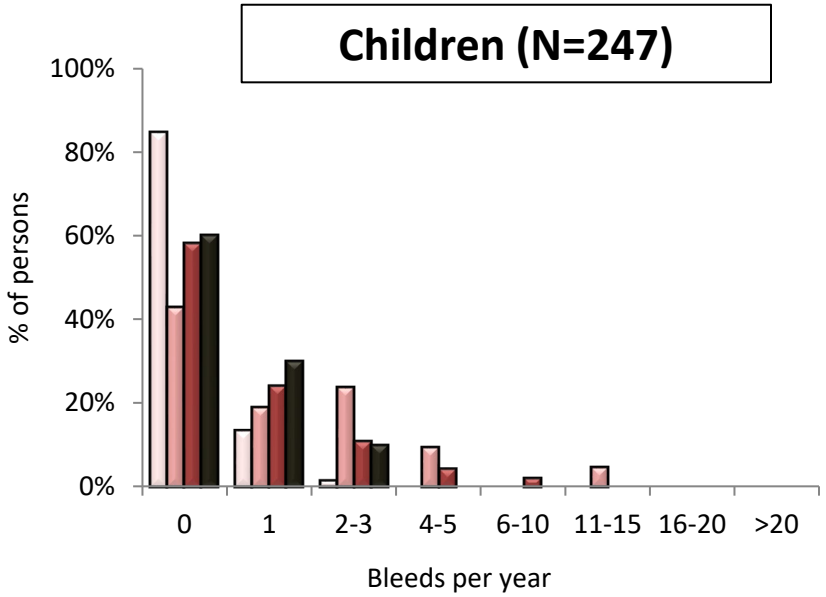
All
Haem A
N=641

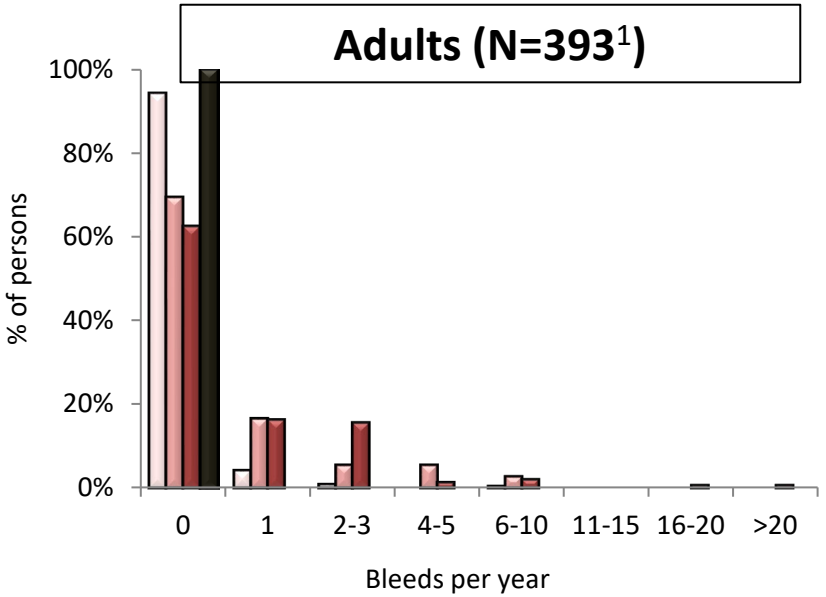
Data from year 2023 – sample size

	Valid persons			Persons with valid annual report			Persons examined			Persons treated	
	N	%		N	%		N	%		N	%
All	641	100%	→	616	96.1%	→	477	74.4%	→	334	52.1%
of them with inhibitor	17			17			17			16	
Children	247	100%	→	247	100.0%	→	221	89.5%	→	135	54.7%
of them with inhibitor	10			10			10			10	
Adults	394	100%	→	369	93.7%	→	256	65.0%	→	199	50.5%
of them with inhibitor	7			7			7			6	

Haem A
N=641

Frequency of bleeding requiring treatment in 2023





Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
125	*21*	*91*	*10*	**N valid**	*210*	*36*	*140*	*7*
0.2	*1.7*	*0.9*	*0.5*	**Mean**	*0.1*	*0.8*	*1.2*	*0.0*
0 (0 – 2)	*1 (0 – 11)*	*0 (0 – 10)*	*0 (0 – 2)*	**Median (min – max)**	*0 (0 – 7)*	*0 (0 – 9)*	*0 (0 – 34)*	*0 (0 – 0)*
106 (84.8%)	*9 (42.9%)*	*53 (58.2%)*	*6 (60%)*	**N (%) with no bleed**	*198 (94.3%)*	*25 (69.4%)*	*88 (62.4%)*	*7 (100%)*

** without inhibitor*

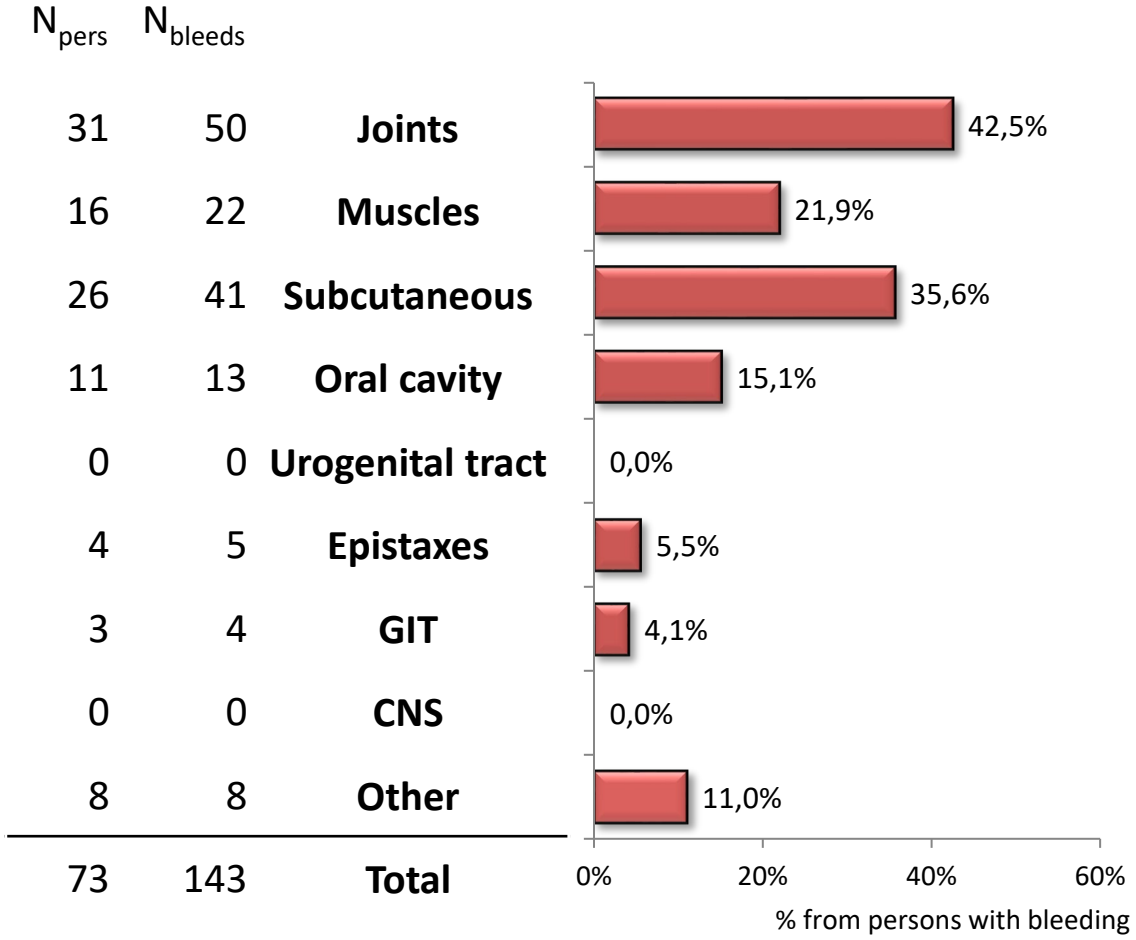
[1] Frequency of bleeding is missing in 1 adult.

Location of bleeds in 2023

Children
Haem A
N=247

73 (29.6%) children experienced bleeding at least once in year; 143 bleeds were recorded in total, 22 bleeds required hospitalization.
All 73 children have recorded location of their bleeds.

174 (70.4%) children recorded no bleed during year 2023.

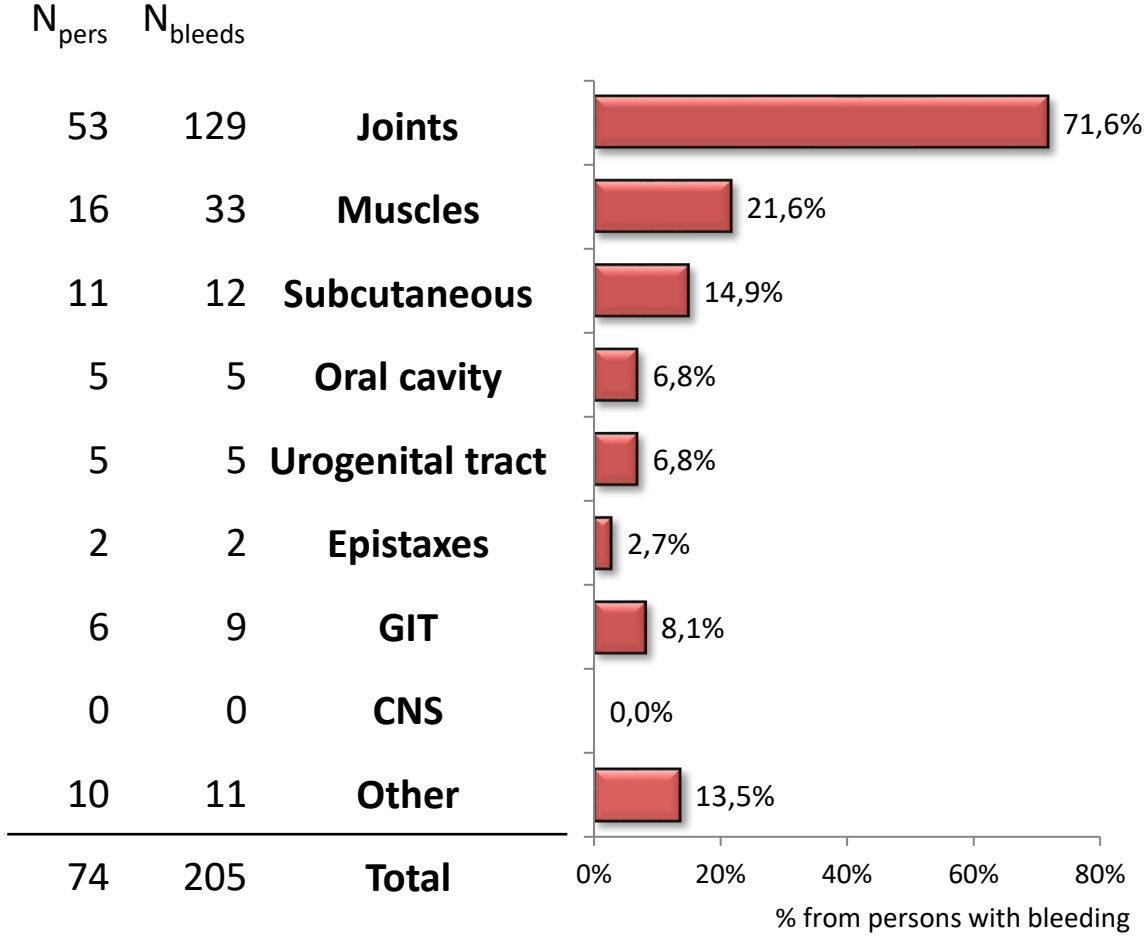


Location of bleeds in 2023

Adults
Haem A
N=393[1]

75 (19.1%) adults experienced bleeding at least once in year; 213 bleeds were recorded in total, 19 bleeds required hospitalization.
74 of these 75 adults have recorded location of their bleeds. Localization is not known in 1 adult.

318 (80.9%) adults have recorded no bleed during year 2023.

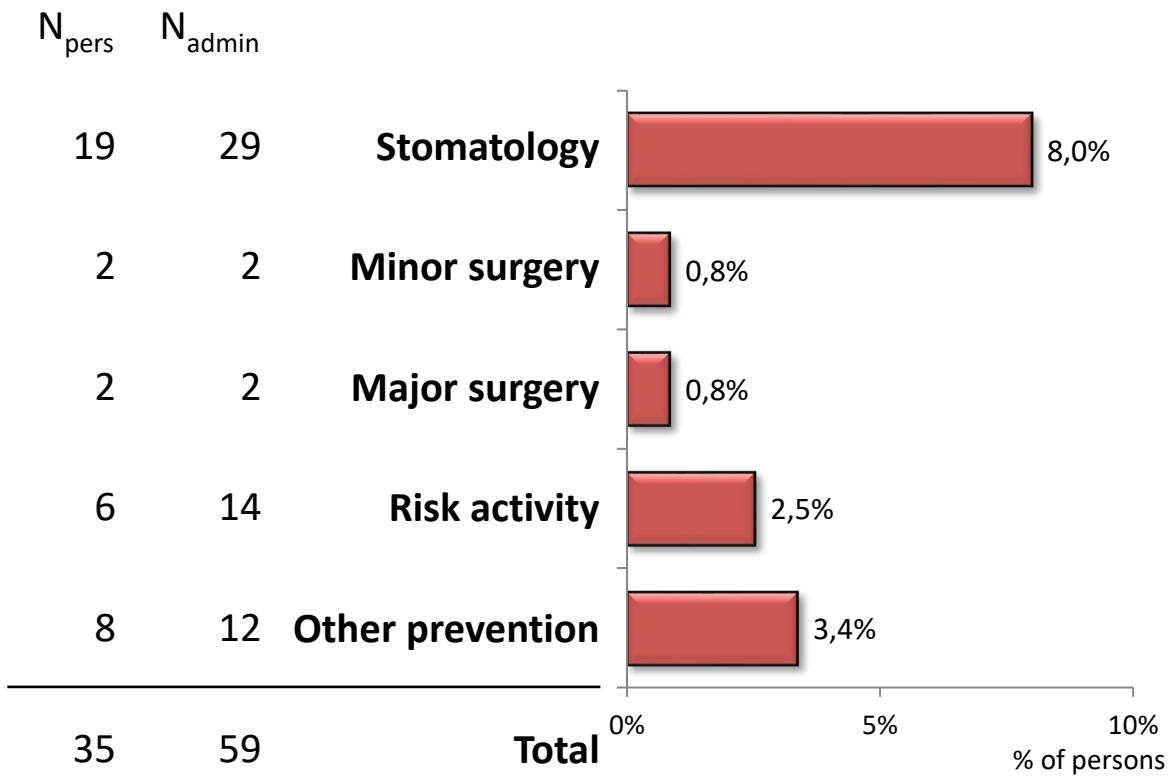


[1]*Frequency of bleeding is missing in 1 adult.*

Preventive administration in 2023

Children
Haem A
N=247

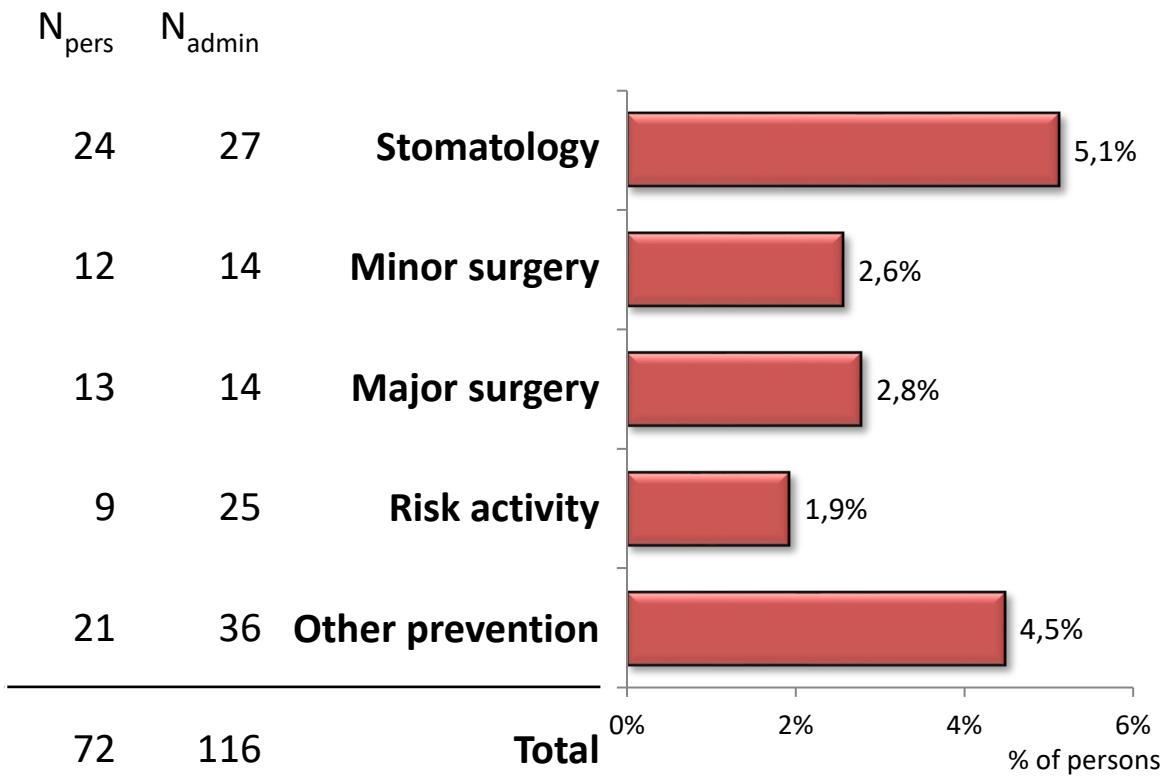
35 (14.2%) children were given factor to prevent bleeding during/before risk situation.
59 preventive administrations were recorded in total.



Preventive administration in 2023

Adults
Haem A
N=394

72 (18.3%) persons were given factor to prevent bleeding during/before risk situation.
116 preventive administ-rations were recorded in total.



ABR according to treatment regimen Haemophilia A without inhibitor

Annual bleeding rate according to treatment regimen

Children Haem A N=237*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*124*	*1*	*13*	*8*	*1*	*90*
Mean	*0.2*	*0.0*	*0.8*	*3.0*	***0.0***	***0.9***
Median (min – max)	*0 (0 – 2)*	*0 (0 – 0)*	*0 (0 – 4)*	*2.5 (0 – 11)*	***0 (0 – 0)***	***0 (0 – 10)***
Total no of recorded bleeds	*21*	*0*	*11*	*24*	***0***	***79***
Children on permanent prophylaxis	***1 (0.8%)***		***8 (38.1%)***		***90 (98.9%)***	
% of factor (FVIII) consumed by children on permanent prophylaxis	***0.7%***		***95.0%***		***100.0%***	

Location of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*124*	*1*	*13*	*8*	*1*	*90*
JOINT BLEEDS						
Mean	*0.1*	*0*	*0.3*	*1.3*	***0.0***	***0.3***
Median (range)	*0 (0 – 2)*	*0 (0 – 0)*	*0 (0 – 3)*	*1 (0 – 4)*	***0 (0 – 0)***	***0 (0 – 3)***
Total no of recorded bleeds	*9*	*0*	*4*	*10*	***0***	***27***
OTHER BLEEDS						
Mean	*0.1*	*0*	*0.5*	*1.8*	***0.0***	***0.6***
Median (range)	*0 (0 – 2)*	*0 (0 – 0)*	*0 (0 – 2)*	*0.5 (0 – 10)*	***0 (0 – 0)***	***0 (0 – 7)***
Total no of recorded bleeds	*13*	*0*	*7*	*14*	***0***	***54***

** without inhibitor*

Treatment regimen:
***OD** = on demand and/or temporary prophylaxis*
***prophy** = permanent prophylaxis (factor or emi)*

Annual bleeding rate according to treatment regimen

Adults
Haem A
N=385*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*210*	*0*	*30*	*6*	*27*	*112*
Mean	*0.1*	*0.0*	*0.8*	*1.0*	***3.7***	***0.6***
Median (min – max)	*0 (0 – 7)*	*(–)*	*0 (0 – 9)*	*1 (0 – 3)*	***1 (0 – 34)***	***0 (0 – 8)***
Total no of recorded bleeds	*21*	*0*	*23*	*6*	***99***	***64***
Adults on permanent prophylaxis	***0 (0%)***		***6 (16.7%)***		***113 (80.7%)***	
% of factor (FVIII) consumed by adults on permanent prophylaxis	***0.0%***		***76.8%***		***92.1%***	

Location of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*210*	*0*	*30*	*6*	*26*	*113*
JOINT BLEEDS						
Mean	*0.1*	*0*	*0.4*	*0.5*	***2.3***	***0.4***
Median (range)	*0 (0 – 7)*	*(–)*	*0 (0 – 4)*	*0 (0 – 2)*	***0 (0 – 29)***	***0 (0 – 5)***
Total no of recorded bleeds	*12*	*0*	*12*	*3*	***61***	***41***
OTHER BLEEDS						
Mean	*0.0*	*0*	*0.4*	*0.5*	***1.1***	***0.2***
Median (range)	*0 (0 – 3)*	*(–)*	*0 (0 – 8)*	*0.5 (0 – 1)*	***0 (0 – 5)***	***0 (0 – 3)***
Total no of recorded bleeds	*9*	*0*	*11*	*3*	***29***	***25***

** without inhibitor; missing frequency of bleeding in 1 adult; missing location of bleeds in 1 adult*

Treatment regimen:
***OD** = on demand and/or temporary prophylaxis*
***prophy** = permanent prophylaxis (factor or emi)*

ABR according to treatment regimen and age

Adults Haem A N=385*

** without inhibitor; missing frequency of bleeding in 1 adult; missing location of bleeds in 1 adult*

Frequency of bleeding	Mild*		Moderate*		Severe*		
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	
N valid	152	0	18	5	21	75	Adults (haem A) born before 1990 N=271
Mean	0.1	0.0	0.5	1.0	**4.3**	**0.5**	
Median (min – max)	0 (0 – 3)	(–)	0 (0 – 5)	1 (0 – 3)	**1 (0 – 34)**	**0 (0 – 5)**	
Total no of recorded bleeds	9	0	9	5	**90**	**38**	
Adults on permanent prophylaxis	**0 (0%)**		**5 (21.7%)**		**76 (78.4%)**		
% of factor (FVIII) consumed by adults on permanent prophylaxis	**0.0%**		**72.1%**		**92.4%**		

Frequency of bleeding	Mild*		Moderate*		Severe*		
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	
N valid	58	0	12	1	6	37	Adults (haem A) born in 1990 or later N=114
Mean	0.2	0.0	1.2	1.0	**1.5**	**0.7**	
Median (min – max)	0 (0 – 7)	(–)	0 (0 – 9)	1 (1 – 1)	**1.5 (0 – 4)**	**0 (0 – 8)**	
Total no of recorded bleeds	12	0	14	1	**9**	**26**	
Adults on permanent prophylaxis	**0 (0%)**		**1 (7.7%)**		**37 (86%)**		
% of factor (FVIII) consumed by adults on permanent prophylaxis	**0.0%**		**86.0%**		**91.4%**		

Joint and other bleeds according to treatment regimen and age

Adults Haem A N=385*

** without inhibitor; missing frequency of bleeding in 1 adult; missing location of bleeds in 1 adult*

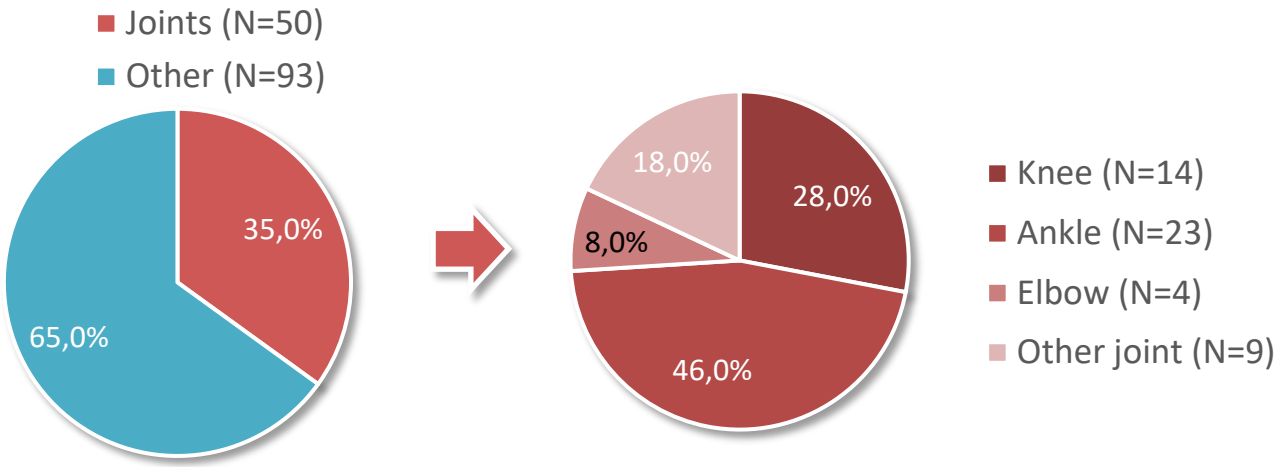
Frequency of bleeding	Mild*		Moderate*		Severe*		
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*	
N valid	*152*	*0*	*18*	*5*	*20*	*76*	
JOINT BLEEDS							
Mean	*0.0*	0	*0.4*	*0.6*	***2.9***	***0.3***	**Adults (haem A) born before 1990 N=271**
Median (range)	*0 (0 – 1)*	(–)	*0 (0 – 4)*	*0 (0 – 2)*	***0 (0 – 29)***	***0 (0 – 3)***	
Total no of recorded bleeds	*3*	0	*7*	*3*	***58***	***25***	
OTHER BLEEDS							
Mean	*0.0*	0	*0.1*	*0.4*	***1.2***	***0.2***	
Median (range)	*0 (0 – 3)*	(–)	*0 (0 – 1)*	*0 (0 – 1)*	***0 (0 – 5)***	***0 (0 – 3)***	
Total no of recorded bleeds	*6*	0	*2*	*2*	***23***	***14***	

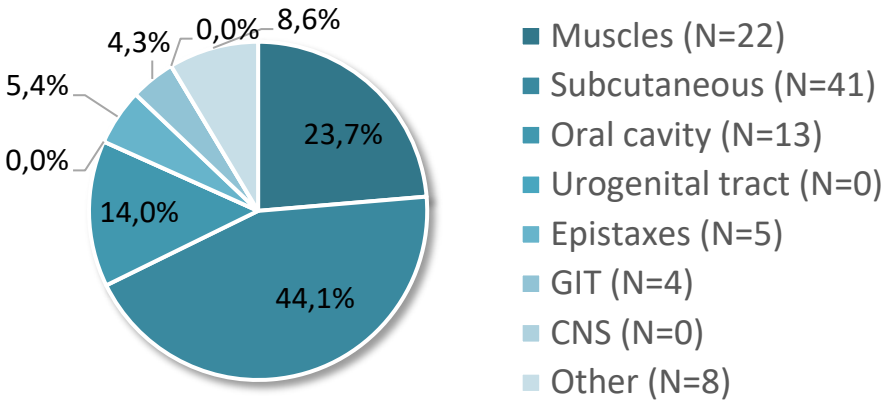
Frequency of bleeding	Mild*		Moderate*		Severe*		
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*	
N valid	*58*	*0*	*12*	*1*	*6*	*37*	
JOINT BLEEDS							
Mean	*0.2*	0	*0.4*	*0.0*	***0.5***	***0.4***	**Adults (haem A) born in 1990 or later N=114**
Median (range)	*0 (0 – 7)*	(–)	*0 (0 – 3)*	*0 (0 – 0)*	***0.5 (0 – 1)***	***0 (0 – 5)***	
Total no of recorded bleeds	*9*	0	*5*	*0*	***3***	***16***	
OTHER BLEEDS							
Mean	*0.1*	0	*0.8*	*1.0*	***1.0***	***0.3***	
Median (range)	*0 (0 – 1)*	(–)	*0 (0 – 8)*	*1 (1 – 1)*	***0.5 (0 – 3)***	***0 (0 – 3)***	
Total no of recorded bleeds	*3*	0	*9*	*1*	***6***	***11***	

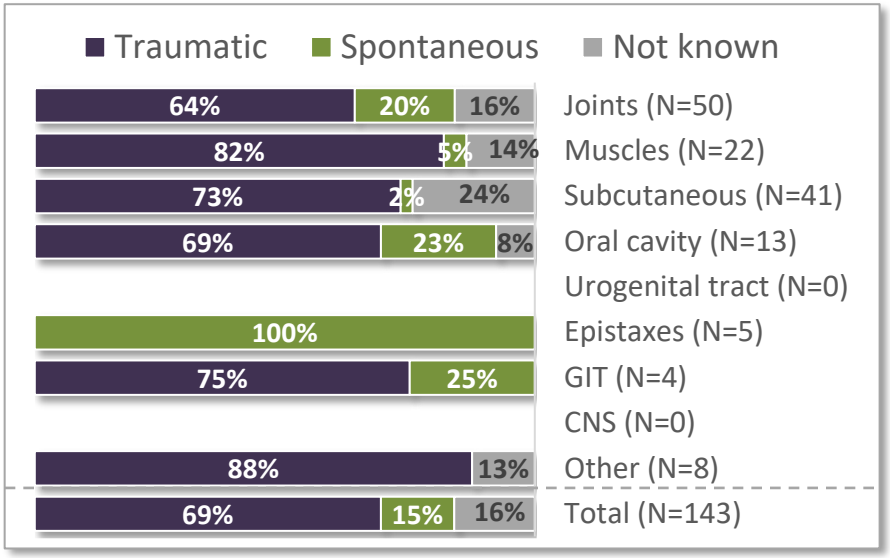
Location and etiology of bleeds

Children Haem A N=143*

** number of bleeds*







Detailed treatment of bleeds

Children Haem A N=143*

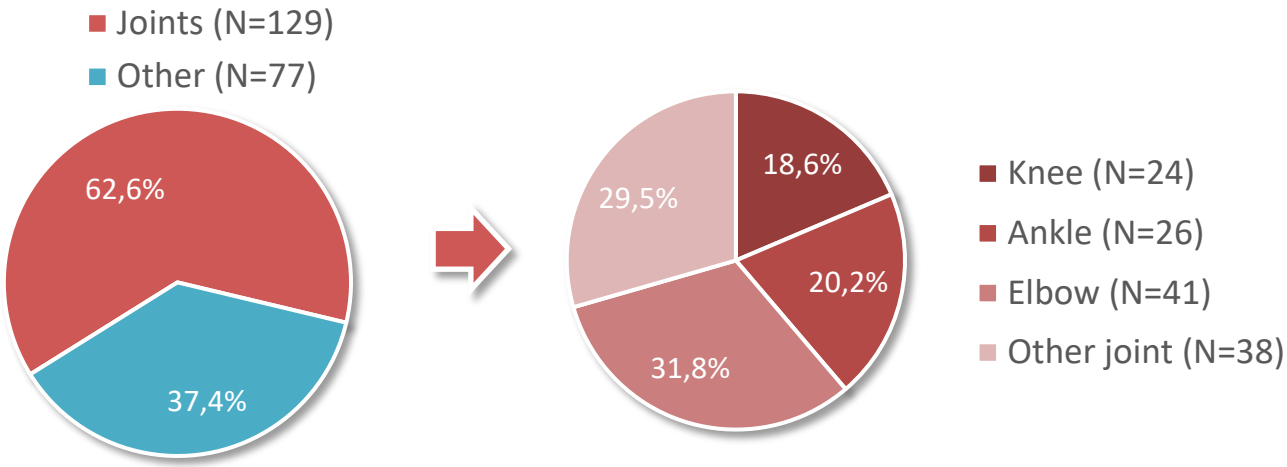
** number of bleeds*

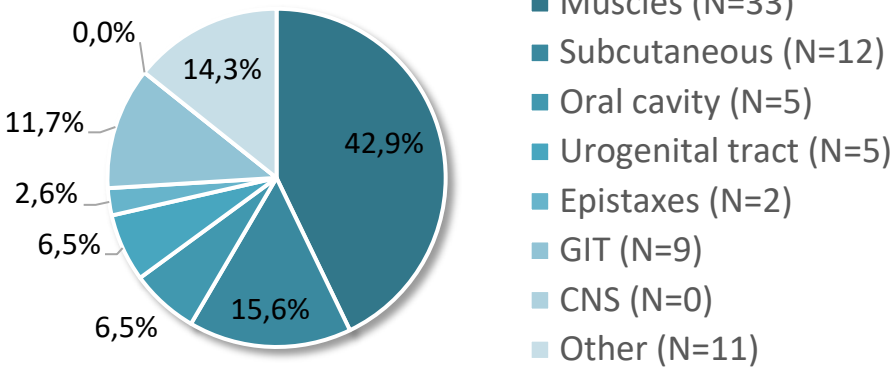
	Joints	Muscles	Subcuta-neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	*50*	*22*	*41*	*13*	*0*	*5*	*4*	*0*	*8*	***143***
FVIII consumption per bleed (IU), valid N	*44*	*20*	*31*	*7*		*2*	*2*		*7*	***113***
geometric mean	3193.6	4129.9	1555.5	959.7		707.1	3240.4		2410.2	**2437.5**
median	**3250.0**	**3000.0**	**1500.0**	**1000.0**		**750.0**	**5750.0**		**2500.0**	**2000.0**
min – max	750–18000	1000–35000	250–9000	500–1500		500–1000	1000–10500		750–7000	**250–35000**
sum	192 000	133 000	66 250	7 000		1 500	11 500		21 250	**432 500**
No. of doses per bleed										
geometric mean	2.1	3.1	1.5	1.1		1.0	2.4		2.5	**1.9**
median	**2**	**3**	**1**	**1**		**0**	**2**		**3**	**1**
min – max	0–14	0–14	0–6	0–2		0–1	0–7		0–5	**0–14**
Duration of therapy per bleed, days										
geometric mean	3.0	3.0	1.8	3.0		2.0	3.6		3.0	**2.6**
median	**3**	**3**	**1**	**3**		**3**	**5**		**2**	**2**
min – max	1–26	1–37	1–21	1–18		1–4	1–7		1–10	**1–37**
N (%) with hospitalization	*6 (12%)*	*3 (13.6%)*	*6 (14.6%)*	*3 (23.1%)*		*0 (0%)*	*2 (50%)*		*2 (25%)*	***22 (15.4%)***
N (%) with rebleeding	*6 (12%)*	*2 (9.1%)*	*6 (14.6%)*	*1 (7.7%)*		*1 (20%)*	*0 (0%)*		*1 (12.5%)*	***17 (11.9%)***

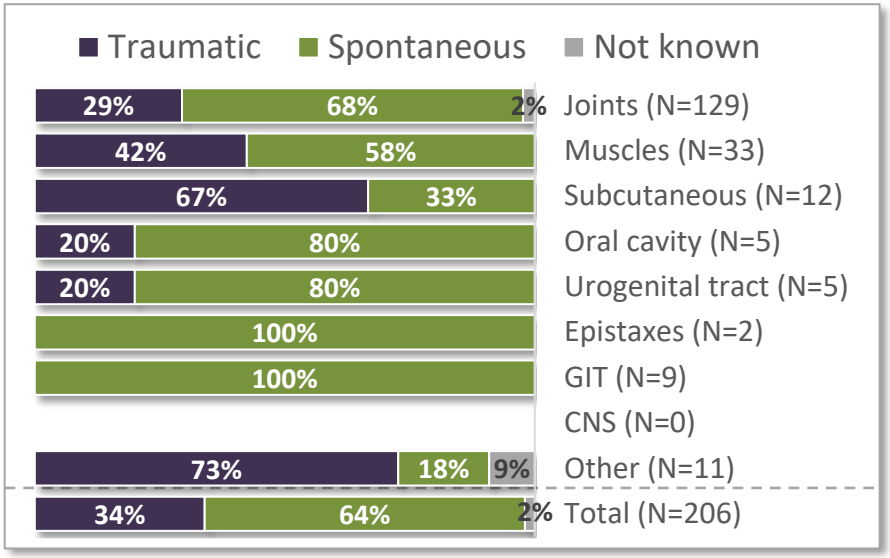
Location and etiology of bleeds

Adults
Haem A
N=206*

** number of bleeds*







Detailed treatment of bleeds

Adults
Haem A
N=206*

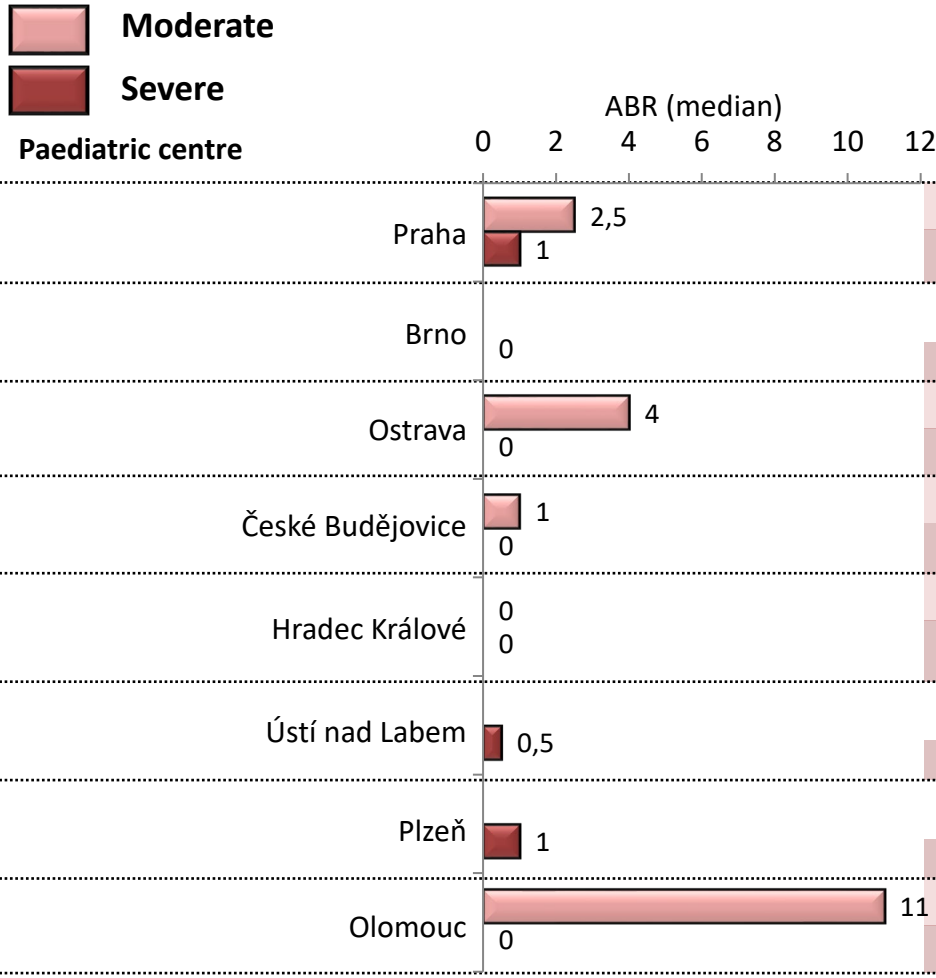
** number of bleeds*

	Joints	Muscles	Subcuta-neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	*129*	*33*	*12*	*5*	*5*	*2*	*9*	*0*	*11*	***206***
FVIII consumption per bleed (IU), valid N	*127*	*33*	*11*	*5*	*5*	*2*	*9*		*10*	***202***
geometric mean	2787.1	2655.7	2942.4	2047.7	5298.5	2449.5	10788.0		9689.8	**3155.1**
median	**3000.0**	**1000.0**	**3000.0**	**2000.0**	**3000.0**	**2500.0**	**11000.0**		**10500.0**	**3000.0**
min – max	500–45000	500–136000	1000–35500	1000–3000	2000–29000	2000–3000	2000–56500		2000–70000	**500–136000**
sum	609 500	406 500	66 500	11 000	48 000	5 000	181 500		178 000	**1 506 000**
No. of doses per bleed										
geometric mean	1.8	2.1	1.6	1.1	2.2	1.4	4.0		4.4	**2.0**
median	**1**	**1**	**1**	**1**	**1**	**2**	**4**		**5**	**1**
min – max	0–45	1–70	0–22	1–2	1–12	1–2	1–14		0–39	**0–70**
Duration of therapy per bleed, days										
geometric mean	1.7	2.0	1.6	1.1	2.2	1.0	4.4		4.6	**1.9**
median	**1**	**1**	**1**	**1**	**1**	**1**	**5**		**6**	**1**
min – max	1–30	1–48	1–10	1–2	1–12	1–1	1–21		1–60	**1–60**
N (%) with hospitalization	*5 (3.9%)*	*4 (12.1%)*	*3 (25%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*	*6 (66.7%)*		*1 (9.1%)*	***19 (9.2%)***
N (%) with rebleeding	*2 (1.6%)*	*0 (0%)*	*1 (8.3%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*	*0 (0%)*		*1 (9.1%)*	***4 (1.9%)***

ABR according to centres Haemophilia A (PWHA)

Annual bleeding rate on permanent prophylaxis

HaemA on prophy
Paed. centres
N=104

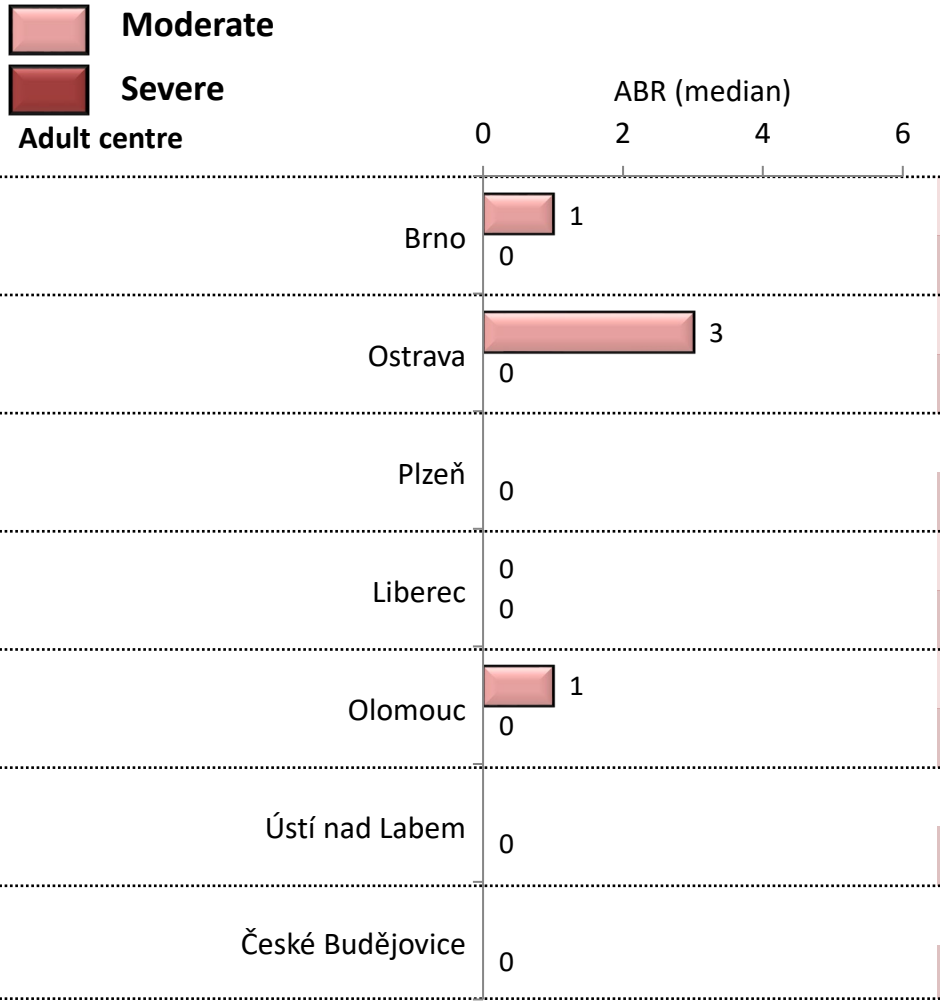


Frequency of bleeding in PWHA without inhibitor on permanent prophylaxis

Paediatric centre	N	Mean	Median	Min	Max	Severity
Praha	4	2.0	**2.5**	0	3	**Moderate**
	36	1.3	**1.0**	0	10	**Severe**
Brno	0					
	26	0.6	**0.0**	0	3	**Severe**
Ostrava	1	4.0	**4.0**	4	4	**Moderate**
	10	0.3	**0.0**	0	1	**Severe**
České Budějovice	1	1.0	**1.0**	1	1	**Moderate**
	11	0.4	**0.0**	0	2	**Severe**
Hradec Králové	1	0.0	**0.0**	0	0	**Moderate**
	3	0.0	**0.0**	0	0	**Severe**
Ústí nad Labem	0					
	4	1.8	**0.5**	0	6	**Severe**
Plzeň	0					
	3	2.0	**1.0**	0	5	**Severe**
Olomouc	1	11.0	**11.0**	11	11	**Moderate**
	3	0.3	**0.0**	0	1	**Severe**

Annual bleeding rate on permanent prophylaxis

HaemA on prophy
Adult centres
N=112

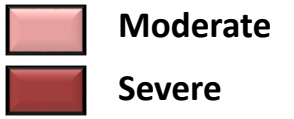


Frequency of bleeding in PWHA without inhibitor on permanent prophylaxis

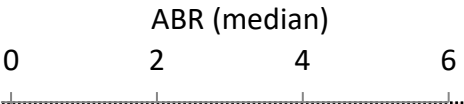
Adult centre	N	Mean	Median	Min	Max	Severity
Brno	2	1.0	**1.0**	1	1	**Moderate**
	34	0.7	**0.0**	0	8	**Severe**
Ostrava	1	3.0	**3.0**	3	3	**Moderate**
	26	0.8	**0.0**	0	5	**Severe**
Plzeň	0					
	16	0.0	**0.0**	0	0	**Severe**
Liberec	2	0.0	**0.0**	0	0	**Moderate**
	5	0.6	**0.0**	0	3	**Severe**
Olomouc	1	1.0	**1.0**	1	1	**Moderate**
	12	0.5	**0.0**	0	2	**Severe**
Ústí nad Labem	0					
	9	0.7	**0.0**	0	3	**Severe**
České Budějovice	0					
	4	0.0	**0.0**	0	0	**Severe**

Annual bleeding rate regardless prophylaxis

HaemA
Paed. centres
N=119



Frequency of bleeding in PWHA without inhibitor regardless of prophylaxis



Paediatric centre	N	Mean	Median	Min	Max	% on permanent prophylaxis
Praha	7	1.4	**2.0**	0	3	**57.1%**
	37	1.3	**1.0**	0	10	**97.3%**
Brno	6	1.3	**1.0**	0	4	**0.0%**
	26	0.6	**0.0**	0	3	**100.0%**
Ostrava	1	4.0	**4.0**	4	4	**100.0%**
	10	0.3	**0.0**	0	1	**100.0%**
České Budějovice	2	0.5	**0.5**	0	1	**50.0%**
	11	0.4	**0.0**	0	2	**100.0%**
Hradec Králové	1	0.0	**0.0**	0	0	**100.0%**
	3	0.0	**0.0**	0	0	**100.0%**
Ústí nad Labem	1	0.0	**0.0**	0	0	**0.0%**
	4	1.8	**0.5**	0	6	**100.0%**
Plzeň	1	1.0	**1.0**	1	1	**0.0%**
	3	2.0	**1.0**	0	5	**100.0%**
Olomouc	2	5.5	**5.5**	0	11	**50.0%**
	4	0.3	**0.0**	0	1	**75.0%**

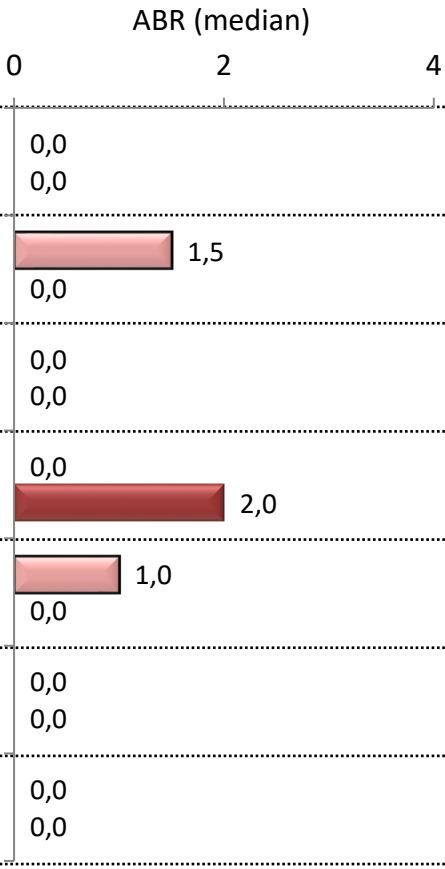
Annual bleeding rate regardless prophylaxis

HaemA
Adult centres
N=169*

** missing ABR in 1 adult*

Moderate
Severe

Frequency of bleeding in PWHA without inhibitor regardless of prophylaxis



Adult centre	N	Mean	Median	Min	Max	% on permanent prophylaxis
Brno	15	0.3	**0.0**	0	1	**13.3%**
	42	0.8	**0.0**	0	8	**81.0%**
Ostrava	6	2.0	**1.5**	0	5	**16.7%**
	27	0.8	**0.0**	0	5	**96.3%**
Plzeň	3	0.7	**0.0**	0	2	**0.0%**
	20	0.7	**0.0**	0	10	**80.0%**
Liberec	3	0.0	**0.0**	0	0	**66.7%**
	10	3.9	**2.0**	0	20	**50.0%**
Olomouc	3	0.7	**1.0**	0	1	**33.3%**
	15	2.7	**0.0**	0	34	**81.3%**
Ústí nad Labem	3	0.0	**0.0**	0	0	**0.0%**
	10	0.7	**0.0**	0	3	**90.0%**
České Budějovice	3	3.0	**0.0**	0	9	**0.0%**
	9	0.6	**0.0**	0	3	**50.0%**

Prophylactic regimens and treatment outcomes

HaemA
Paed. centres
N=119

Paediatric centre	Severity	Total N	PERMANENT PROPHYLAXIS										ON-DEMAND / TEMPORARY PROPHY		
			% of patients	N	Dosing of prophylaxis SHL (IU/kg per week)		Dosing of prophylaxis EHL (IU/kg per week)		Dosing of EMI prophylaxis (mg/kg per week)		ABR		N	ABR	
					Mean	Median	Mean	Median	Mean	Median	Mean	Median		Mean	Median
Praha	Moderate	7	**57.1%**	4	84.3	**84.3**	60.4	**60.4**			2.0	**2.5**	3	0.7	**0.0**
	Severe	37	**97.3%**	36	82.7	**93.8**	68.3	**68.4**	1.6	**1.6**	1.3	**1.0**	1	1.0	**1.0**
Brno	Moderate	6	**0.0%**	0									6	1.3	**1.0**
	Severe	26	**100.0%**	26			76.5	**88.8**	1.2	**1.1**	0.6	**0.0**	0		
Ostrava	Moderate	1	**100.0%**	1	70.1	**70.1**	75.0	**75.0**			4.0	**4.0**	0		
	Severe	10	**100.0%**	10			76.4	**76.4**	1.4	**1.5**	0.3	**0.0**	0		
Č. Budějovice	Moderate	2	**50.0%**	1							1.0	**1.0**	1	0.0	**0.0**
	Severe	11	**100.0%**	11	111.1	**111.1**	68.8	**69.1**			0.4	**0.0**	0		
Hradec Králové	Moderate	1	**100.0%**	1			61.4	**61.4**			0.0	**0.0**	0		
	Severe	3	**100.0%**	3			99.5	**91.0**			0.0	**0.0**	0		
Ústí nad Labem	Moderate	1	**0.0%**	0									1	0.0	**0.0**
	Severe	4	**100.0%**	4			52.3	**52.3**	1.3	**1.3**	1.8	**0.5**	0		
Plzeň	Moderate	1	**0.0%**	0									1	1.0	**1.0**
	Severe	3	**100.0%**	3			12.4	**12.4**	0.8	**0.8**	2.0	**1.0**	0		
Olomouc	Moderate	2	**50.0%**	1					2.5	**2.5**	11.0	**11.0**	1	0.0	**0.0**
	Severe	4	**75.0%**	3			35.7	**35.7**	2.0	**1.4**	0.3	**0.0**	1	0.0	**0.0**

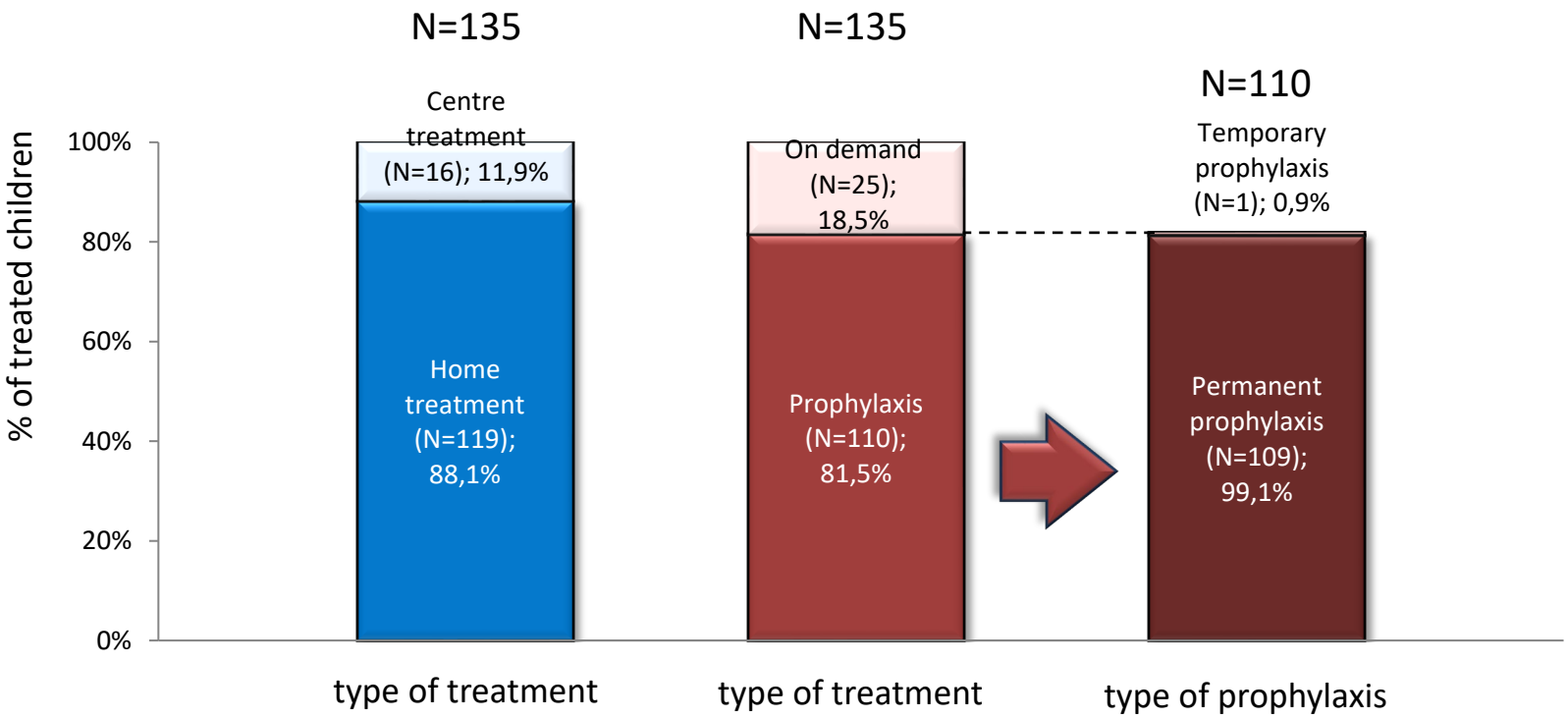
Prophylactic regimens and treatment outcomes

HaemA
Adult centres
N=170

Adult centre	Severity	Total N	PERMANENT PROPHYLAXIS											ON-DEMAND / TEMPORARY PROPHY			
			% of patients	N	Dosing of prophylaxis SHL (IU/kg per week)		Dosing of prophylaxis EHL (IU/kg per week)		Dosing of EMI prophylaxis (mg/kg per week)		ABR		Age	N	ABR		Age
					Mean	Median	Mean	Median	Mean	Median	Mean	Median	Median		Mean	Median	Median
Brno	Moderate	15	**13.3%**	2			62.0	**62.0**			1.0	**1.0**	54	13	0.2	**0.0**	39
	Severe	42	**81.0%**	34	88.9	**70.5**	66.1	**64.2**	1.4	**1.4**	0.7	**0.0**	38	8	1.1	**1.0**	51
Ostrava	Moderate	6	**16.7%**	1			51.7	**51.7**			3.0	**3.0**	71	5	1.8	**0.0**	34
	Severe	27	**96.3%**	26	72.6	**66.4**	76.2	**74.1**	1.4	**1.5**	0.8	**0.0**	44	1	0.0	**0.0**	51
Plzeň	Moderate	3	**0.0%**	0										3	0.7	**0.0**	47
	Severe	20	**80.0%**	16	52.2	**53.0**	59.9	**51.9**	1.5	**1.5**	0.0	**0.0**	45	4	3.3	**1.5**	59
Liberec	Moderate	3	**66.7%**	2	56.3	56.3			1.6	**1.6**	0.0	**0.0**	50	1	0.0	**0.0**	42
	Severe	10	**50.0%**	5			78.0	**71.9**			0.6	**0.0**	30	5	7.2	**4.0**	68
Olomouc	Moderate	3	**33.3%**	1	72.3	**72.3**					1.0	**1.0**	24	2	0.5	**0.5**	24
	Severe	16	**81.3%**	13	56.1	**54.2**	50.1	**48.8**	2.2	**2.2**	0.5	**0.0**	37	3	11.3	**0.0**	63
Ústí n. Labem	Moderate	3	**0.0%**	0										3	0.0	**0.0**	26
	Severe	10	**90.0%**	9			66.9	**66.7**	1.4	**1.4**	0.7	**0.0**	39	1	1.0	**1.0**	44
Č. Budějovice	Moderate	3	**0.0%**	0										3	3.0	**0.0**	70
	Severe	9	**50.0%**	4	300.0	**300.0**	70.2	**61.5**			0.0	**0.0**	55	4	1.3	**1.0**	55

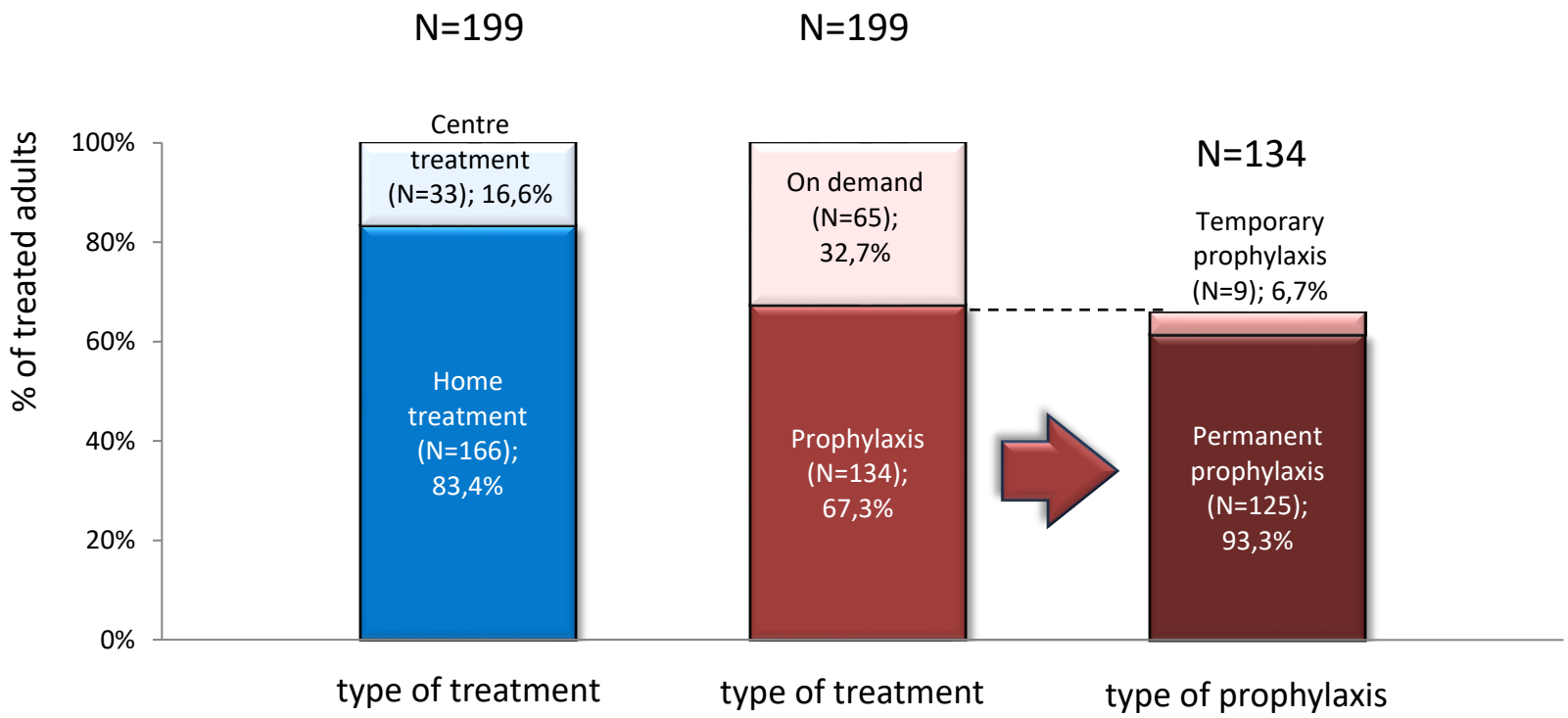
Children
Haem A
N=135

Type of treatment (subgroup of treated patients)



Adults
Haem A
N=199

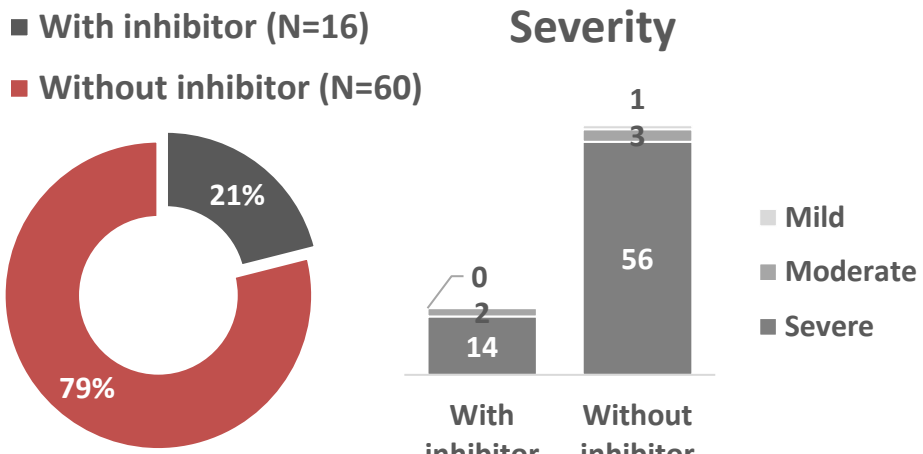
Type of treatment (subgroup of treated patients)

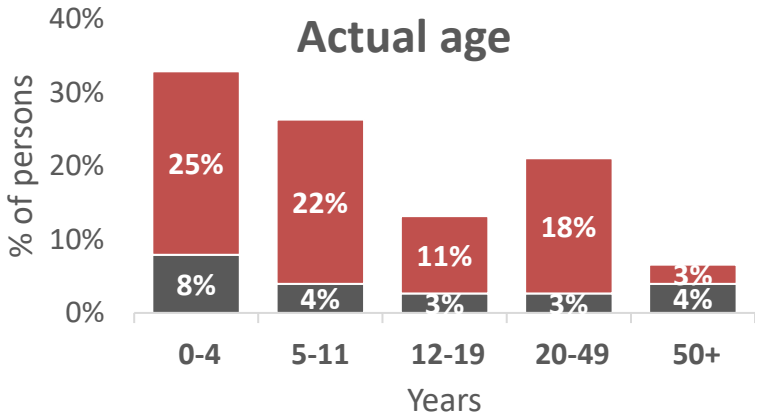


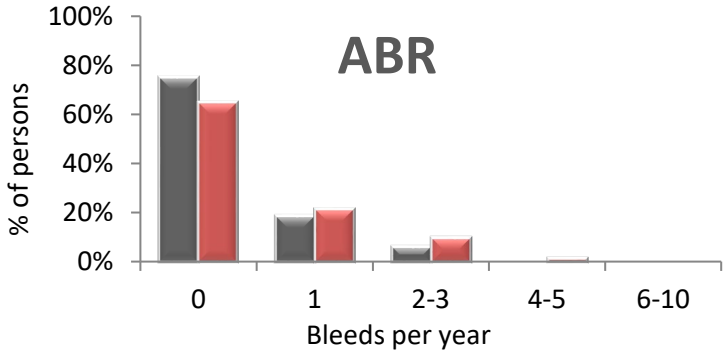
Emicizumab treatment in 2023

Haem A
N=76[1]

[1] *patients on emicizumab prophylaxis in a given year*







	Total	With inhibitor	Without inhibitor
N valid	*76*	*16*	*60*
ABR			
Mean	*0.63*	*0.31*	*0.72*
Median (min – max)	*0 (0 – 11)*	*0 (0 – 2)*	*0 (0 – 11)*
N (%) with no bleed	*51 (67%)*	*12 (75%)*	*39 (65%)*
EMI DOSE (mg/kg/week)			
Mean	*1.47*	*1.50*	*1.46*
Median (min – max)	*1.5 (0.8 – 3.2)*	*1.5 (1.1 – 2.9)*	*1.5 (0.8 – 3.2)*
EMI FREQUENCY			
Once a week	*7 (9.2%)*	*5 (31.3%)*	*2 (3.3%)*
Once every 2 weeks	*51 (67.1%)*	*10 (62.5%)*	*41 (68.3%)*
Once a month	*2 (2.6%)*	*0 (0%)*	*2 (3.3%)*
Other, NA	*16 (21.1%)*	*1 (6.2%)*	*15 (25%)*

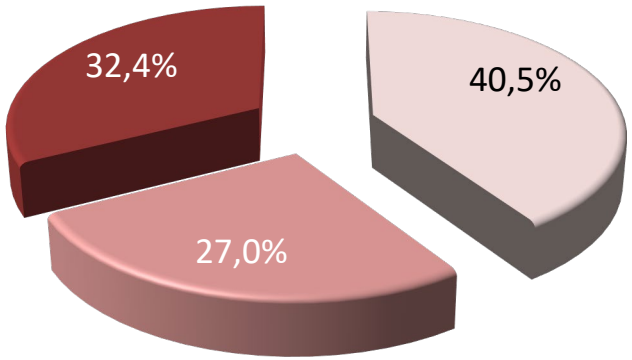
Demographic characteristics Haemophilia B

Severity of haemophilia B

Haem B
N=104

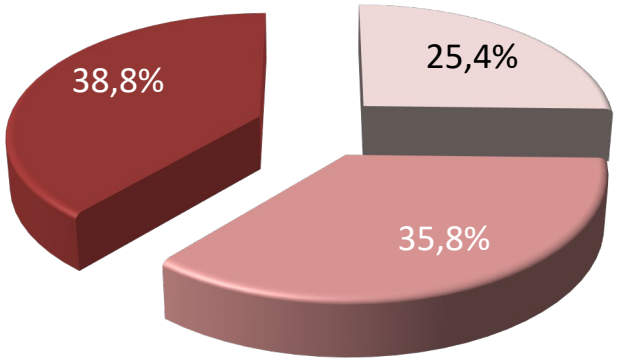
Children (N=37)

- Mild (N=15)
- Moderate (N=10)
- Severe (N=12)



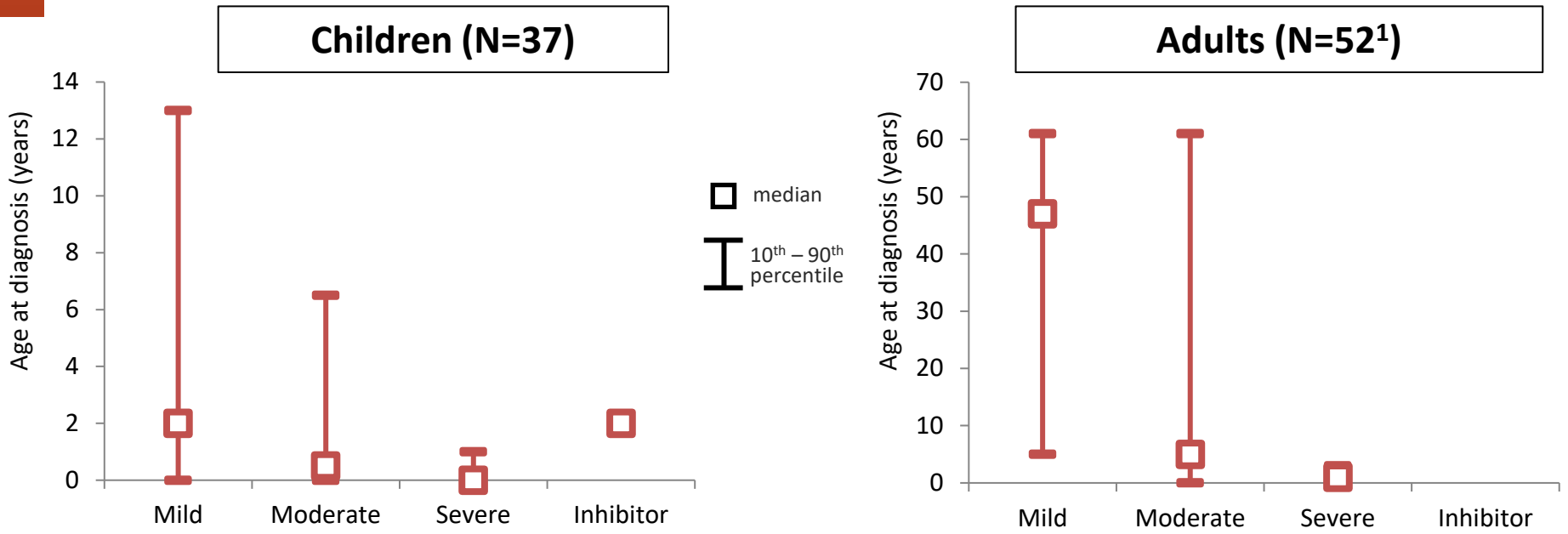
Adults (N=67)

- Mild (N=17)
- Moderate (N=24)
- Severe (N=26)



Age at diagnosis according to severity of haemophilia B

Haem B
N=104



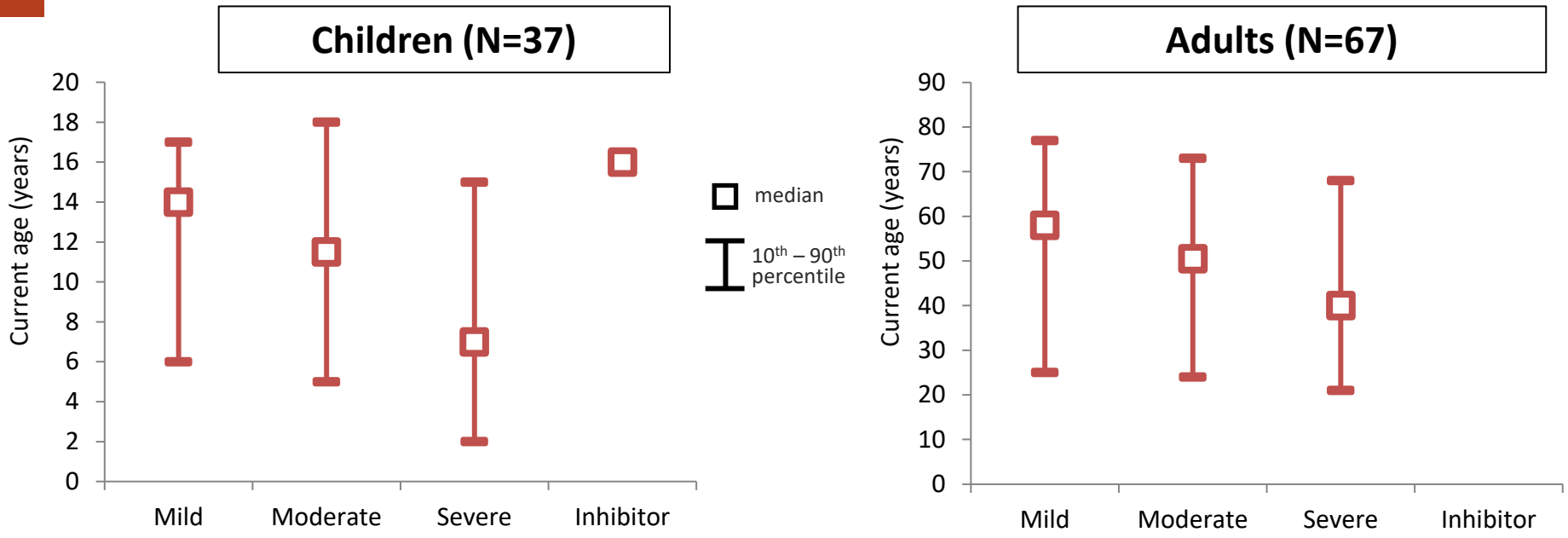
Mild*	Moderate*	Severe*	Inhibitor+	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor+
15	*10*	*12*	*1*	**N valid**	*11*	*19*	*22*	*0*
4.3	*2.1*	*0.4*	*2.0*	**Mean**	*34.1*	*11.9*	*1.3*	
2 (0 – 16)	*0.5 (0 – 8)*	*0 (0 – 2)*	*2 (2 – 2)*	**Median (min – max)**	*47 (2 – 67)*	*5 (0 – 69)*	*1 (0 – 4)*	

[1] *Missing information on year of diagnosis in 15 adults.*

** including persons with inhibitor*
+ in 2023

Haem B
N=104

Actual age according to severity of haemophilia B



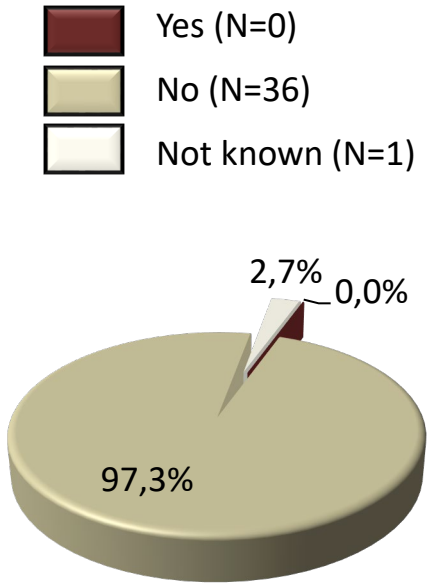
Mild*	Moderate*	Severe*	Inhibitor+	Current age++ (years)	Mild*	Moderate*	Severe*	Inhibitor+
15	*10*	*12*	*1*	**N valid**	*17*	*24*	*26*	*0*
12.2	*12.0*	*7.5*	*16.0*	**Mean**	*51.5*	*50.5*	*43.6*	
14 (5 – 17)	*11.5 (4 – 18)*	*7 (1 – 16)*	*16 (16 – 16)*	**Median (min – max)**	*58 (19 – 85)*	*50.5 (19 – 84)*	*40 (19 – 72)*	

** including persons with inhibitor*
+ in 2023
++ age reached in year 2023

Hepatitis (ever) experienced

Children
Haem B
N=37

Experienced hepatitis



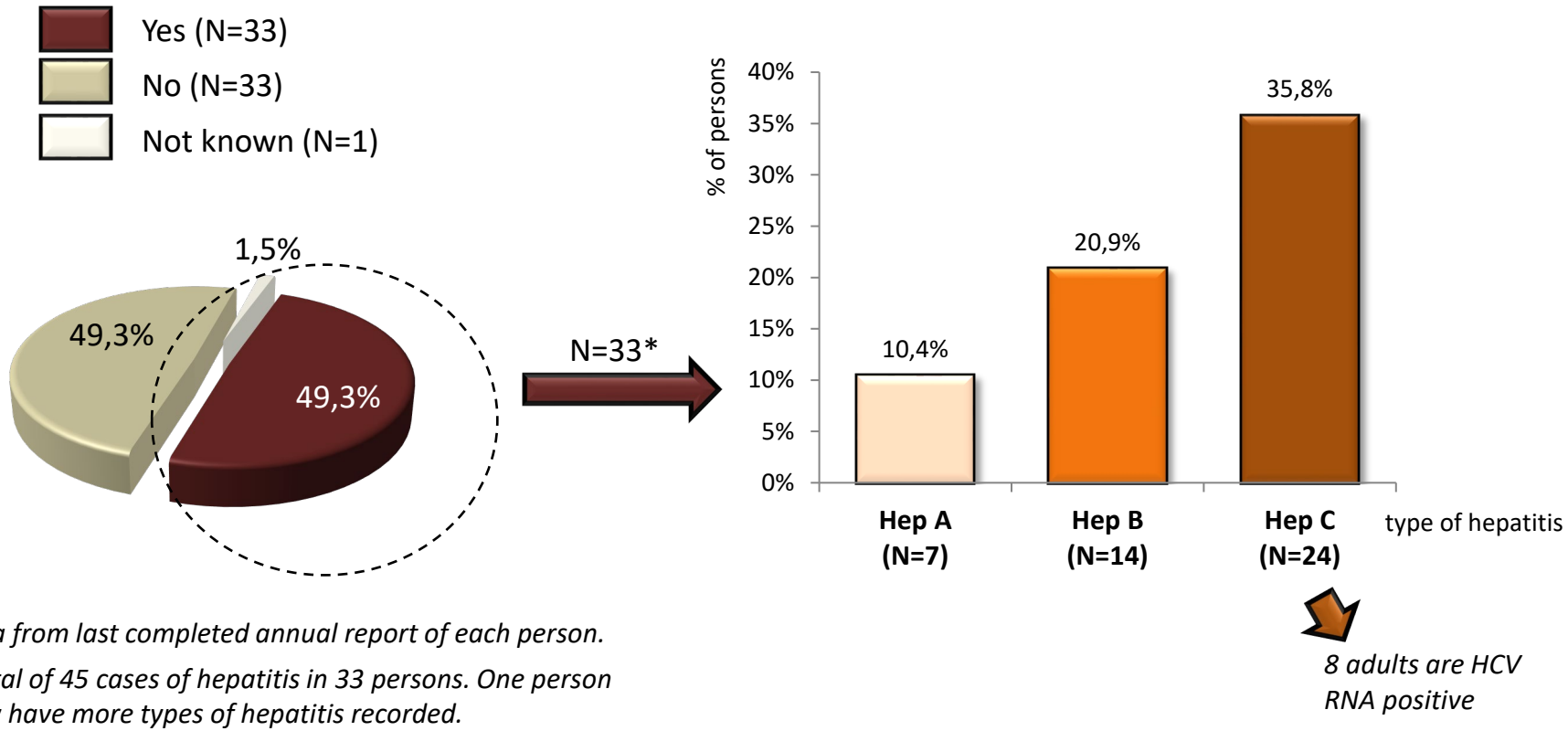
No child has hepatitis.

Data from last completed annual report of each person.

Hepatitis (ever) experienced

Adults
Haem B
N=67

Experienced hepatitis



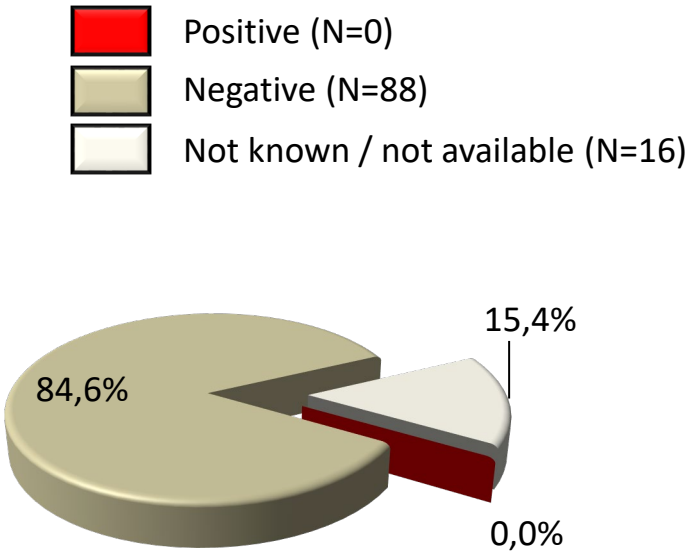
Data from last completed annual report of each person.

**Total of 45 cases of hepatitis in 33 persons. One person may have more types of hepatitis recorded.*

HIV

All
Haem B
N=104

HIV



No HIV-positive person.

Data from last completed annual report of each person.

Treatment outcomes and bleeding frequency Haemophilia B

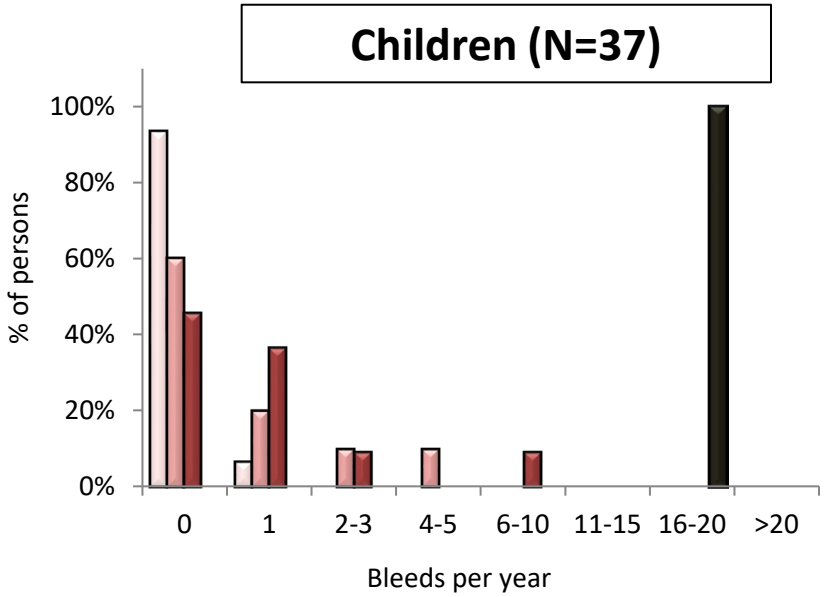
All
Haem B
N=104

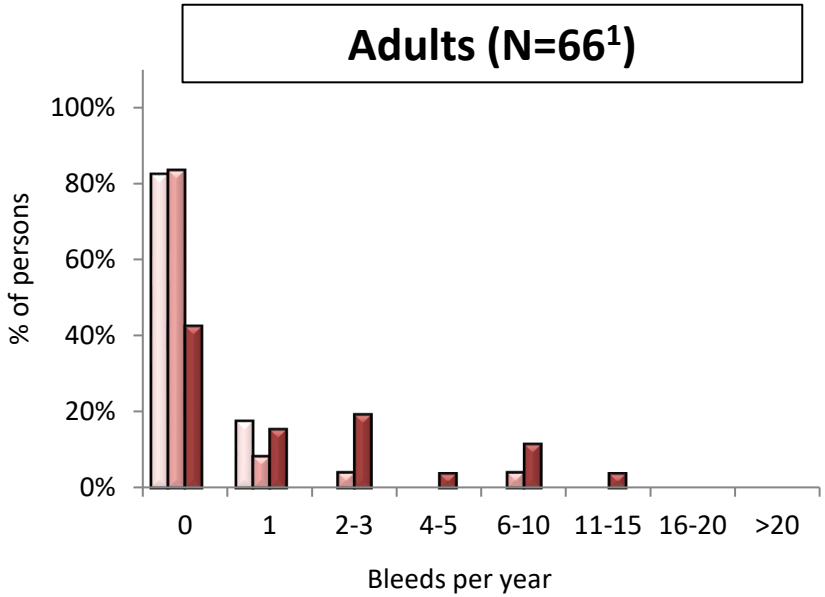
Data from year 2023 – sample size

	Valid persons			Persons with valid annual report			Persons examined			Persons treated	
	N	%		N	%		N	%		N	%
All	104	100%	→	99	95.2%	→	84	80.8%	→	61	58.7%
of them with inhibitor	1			1			1			1	
Children	37	100%	→	37	100.0%	→	35	94.6%	→	19	51.4%
of them with inhibitor	1			1			1			1	
Adults	67	100%	→	62	92.5%	→	49	73.1%	→	42	62.7%
of them with inhibitor	0			0			0			0	

Haem B
N=103[1]

Frequency of bleeding requiring treatment in 2023





Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
15	*10*	*11*	*1*	**N valid**	*17*	*24*	*25*	*0*
0.1	*1.0*	*1.4*	*16.0*	**Mean**	*0.2*	*0.5*	*2.0*	
0 (0 – 1)	*0 (0 – 5)*	*1 (0 – 9)*	*16 (16 – 16)*	**Median (min – max)**	*0 (0 – 1)*	*0 (0 – 6)*	*1 (0 – 11)*	
14 (93.3%)	*6 (60%)*	*5 (45.5%)*	*0 (0%)*	**N (%) with no bleed**	*14 (82.4%)*	*20 (83.3%)*	*11 (42.3%)*	

** without inhibitor*

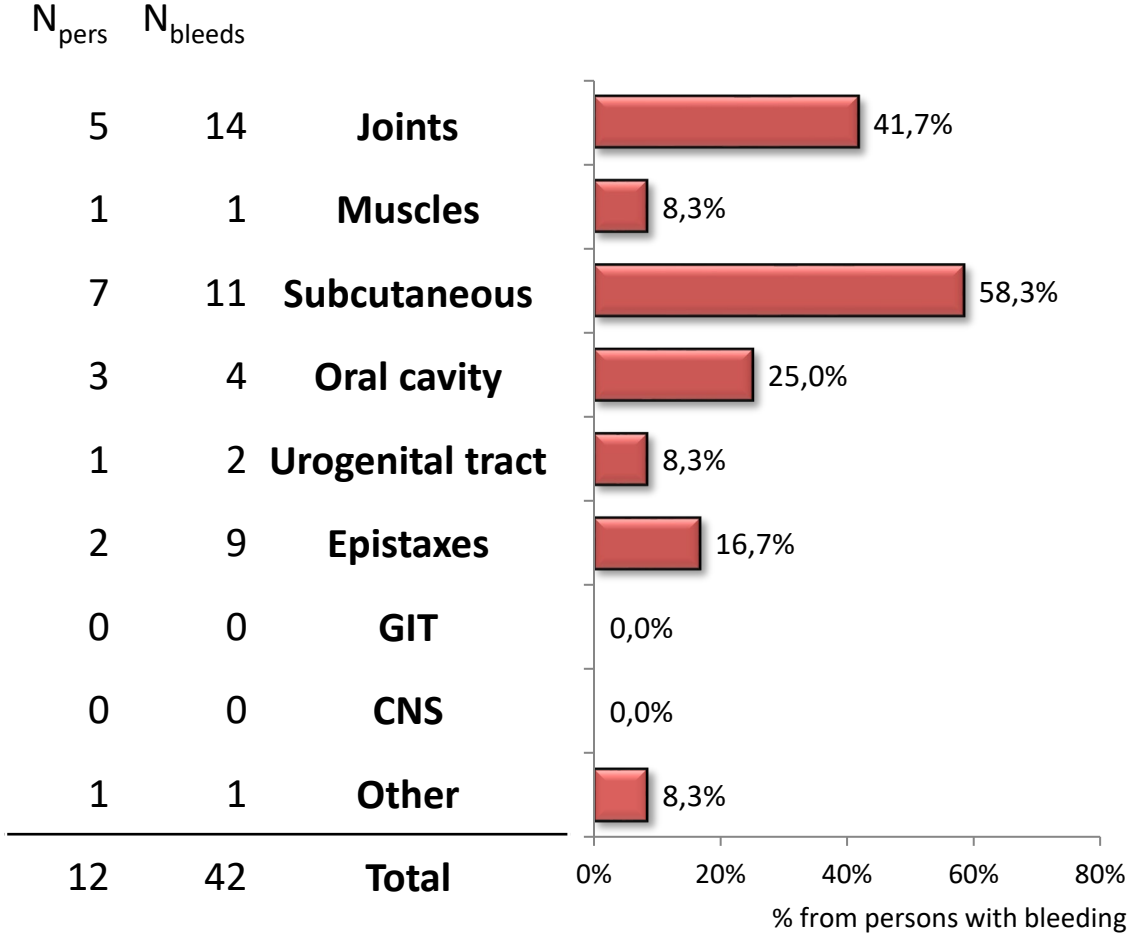
[1]Frequency of bleeding is missing in 1 adult.

Location of bleeds in 2023

Children
Haem B
N=37

12 (32.4%) children experienced bleeding at least once in year; 42 bleeds were recorded in total, 5 bleeds required hospitalization.
All of these 12 children have recorded location of their bleeds.

25 (67.6%) children recorded no bleed during year 2023.



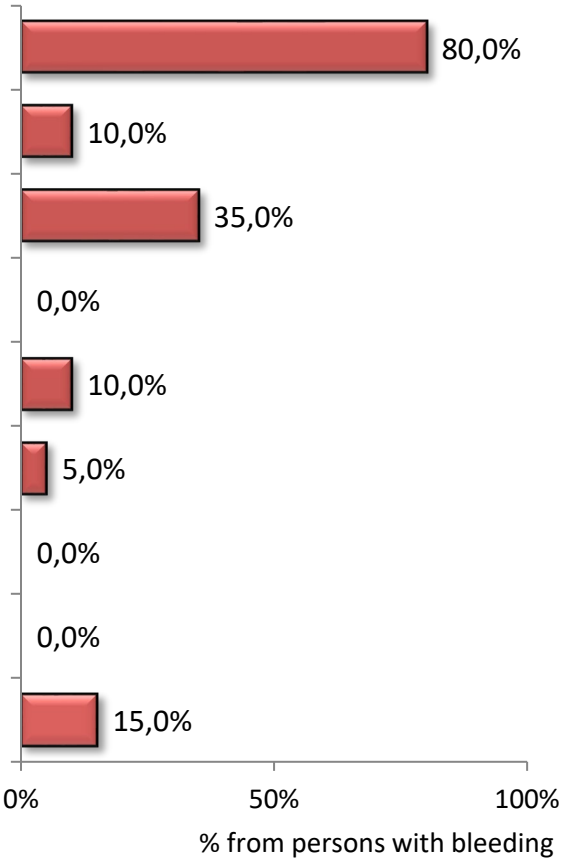
Location of bleeds in 2023

Adults
Haem B
N=66[1]

21 (31.8%) adults experienced bleeding requiring treatment at least once in year; 64 bleeds were recorded in total, 7 bleeds required hospitalization.
20 of these 21 adults have recorded location of their bleeds.

45 (68.2%) adults have recorded no bleed during year 2023.

N_{pers}	N_{bleeds}		
16	39	**Joints**	80,0%
2	2	**Muscles**	10,0%
7	8	**Subcutaneous**	35,0%
0	0	**Oral cavity**	0,0%
2	4	**Urogenital tract**	10,0%
1	1	**Epistaxes**	5,0%
0	0	**GIT**	0,0%
0	0	**CNS**	0,0%
3	6	**Other**	15,0%
20	58	**Total**	

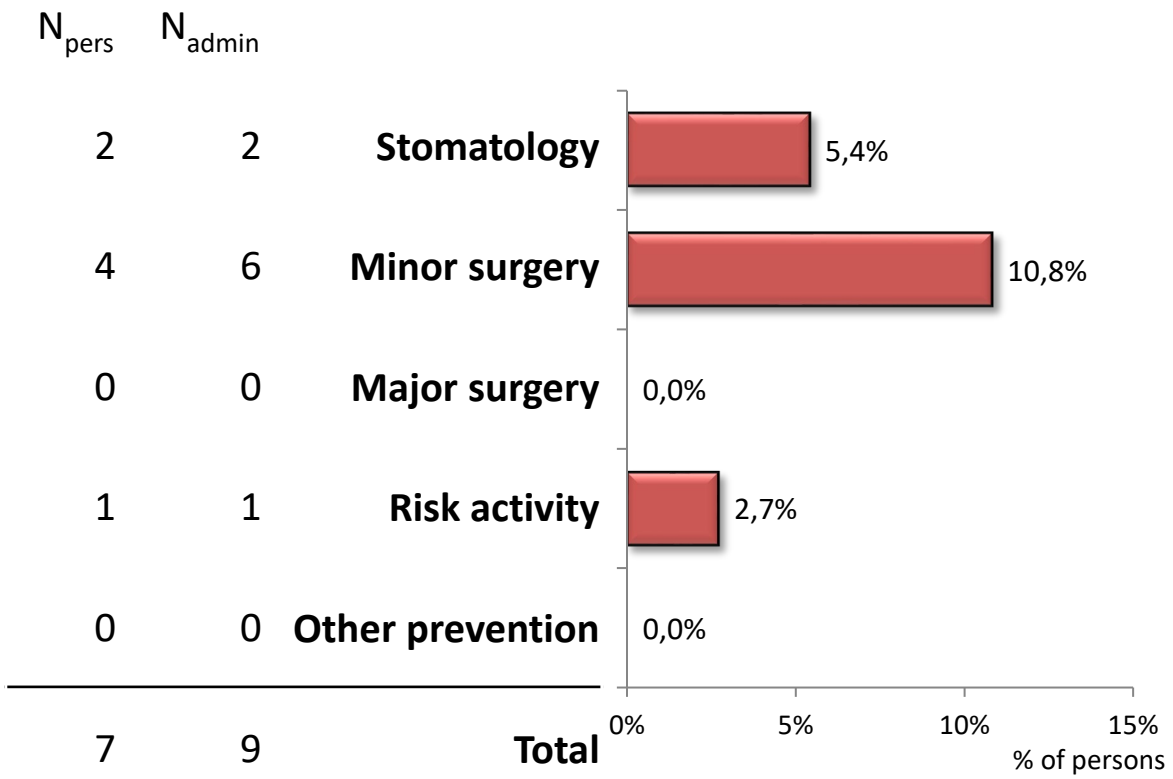


[1]*Frequency of bleeding is missing in 1 adult.*

Preventive administration in 2023

Children
Haem B
N=37

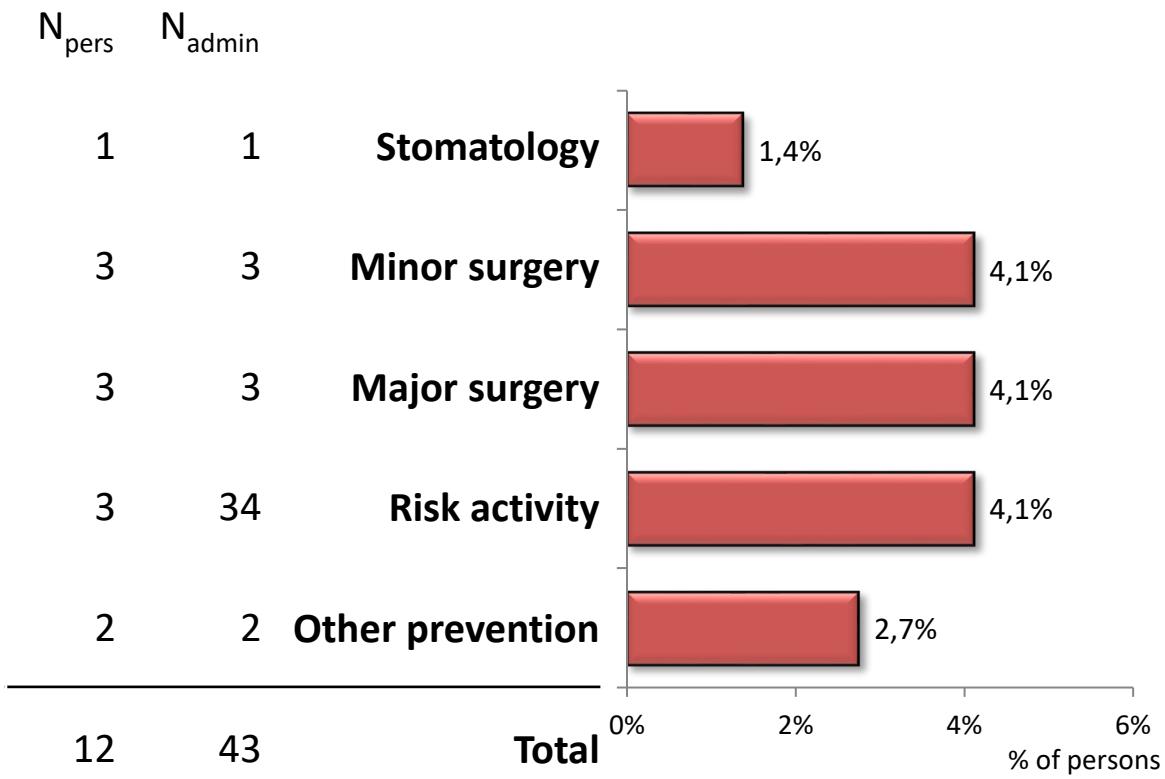
7 (18.9%) children were given factor to prevent bleeding during/before risk situation.
9 preventive administrations were recorded in total.



Preventive administration in 2023

Adults
Haem B
N=67

12 (17.9%) persons were given factor to prevent bleeding during/before risk situation.
43 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=36*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*15*	*0*	*8*	*2*	*3*	*8*
Mean	*0.1*	*0.0*	*0.5*	*3.0*	***1.0***	***1.5***
Median (min – max)	*0 (0 – 1)*	*(–)*	*0 (0 – 3)*	*3 (1 – 5)*	***1 (0 – 2)***	***0.5 (0 – 9)***
Total no of recorded bleeds	*1*	*0*	*4*	*6*	***3***	***12***
Children on permanent prophylaxis	***0 (0%)***		***2 (20%)***		***8 (72.7%)***	
% of factor (FVIII) consumed by children on permanent prophylaxis	***0.0%***		***88.7%***		***96.4%***	

Location of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*15*	*0*	*8*	*2*	*3*	*8*
JOINT BLEEDS						
Mean	*0.1*	*0*	*0.1*	*1.5*	***0.0***	***0.1***
Median (range)	*0 (0 – 1)*	*(–)*	*0 (0 – 1)*	*1.5 (0 – 3)*	***0 (0 – 0)***	***0 (0 – 1)***
Total no of recorded bleeds	*1*	*0*	*1*	*3*	***0***	***1***
OTHER BLEEDS						
Mean	*0.0*	*0*	*0.4*	*1.5*	***1.0***	***1.4***
Median (range)	*0 (0 – 0)*	*(–)*	*0 (0 – 2)*	*1.5 (1 – 2)*	***1 (0 – 2)***	***0 (0 – 9)***
Total no of recorded bleeds	*0*	*0*	*3*	*3*	***3***	***11***

** without inhibitor*

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

Annual bleeding rate according to treatment regimen

Adults
Haem B
N=66*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*17*	*0*	*19*	*5*	*6*	*19*
Mean	*0.2*	*0.0*	*0.5*	*0.2*	***1.3***	***2.2***
Median (min – max)	*0 (0 – 1)*	*(–)*	*0 (0 – 6)*	*0 (0 – 1)*	***0 (0 – 5)***	***1 (0 – 11)***
Total no of recorded bleeds	*3*	*0*	*10*	*1*	***8***	***42***
Adults on permanent prophylaxis	***0 (0%)***		***5 (20.8%)***		***19 (73.1%)***	
% of factor (FVIII) consumed by children on permanent prophylaxis	***0.0%***		***85.4%***		***90.1%***	

Location of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*
N valid	*17*	*0*	*19*	*5*	*6*	*19*
JOINT BLEEDS						
Mean	*0.0*	*0*	*0.3*	*0.0*	***1.0***	***1.4***
Median (range)	*0 (0 – 0)*	*(–)*	*0 (0 – 3)*	*0 (0 – 0)*	***0 (0 – 3)***	***1 (0 – 6)***
Total no of recorded bleeds	*0*	*0*	*6*	*0*	***6***	***27***
OTHER BLEEDS						
Mean	*0.2*	*0*	*0.2*	*0.2*	***0.3***	***0.6***
Median (range)	*0 (0 – 1)*	*(–)*	*0 (0 – 4)*	*0 (0 – 1)*	***0 (0 – 2)***	***0 (0 – 5)***
Total no of recorded bleeds	*3*	*0*	*4*	*1*	***2***	***11***

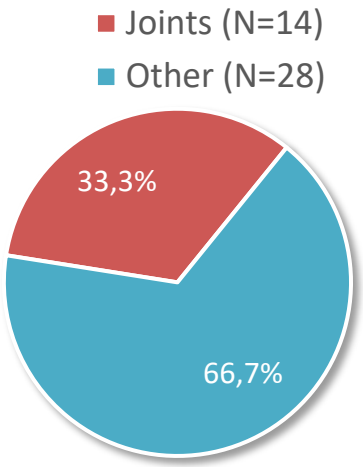
** without inhibitor; missing frequency of bleeding in 1 adult*

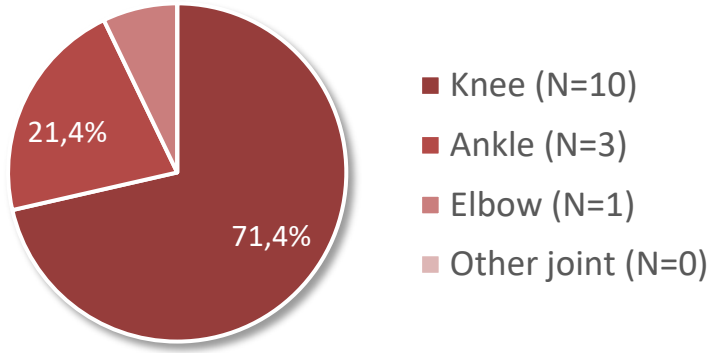
Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

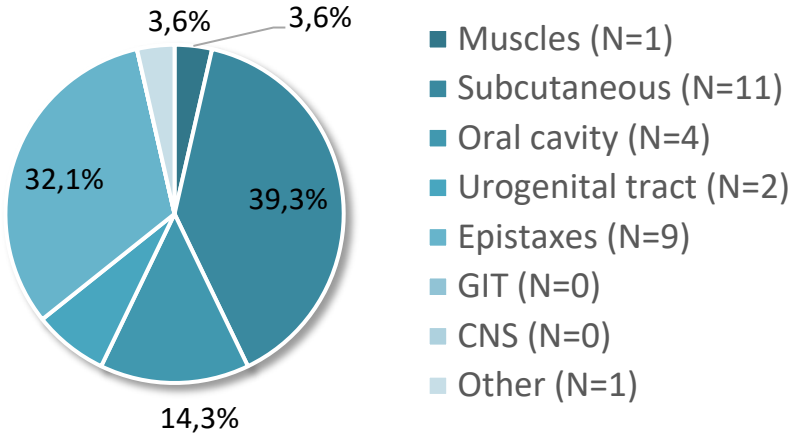
Children
Haem B
N=42*

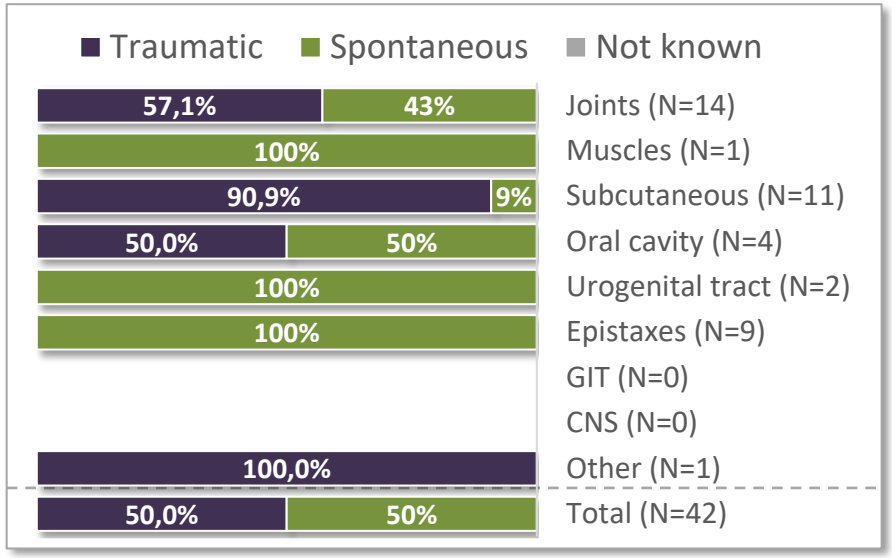
Location and etiology of bleeds

** number of bleeds*









Detailed treatment of bleeds

Children Haem B N=42*

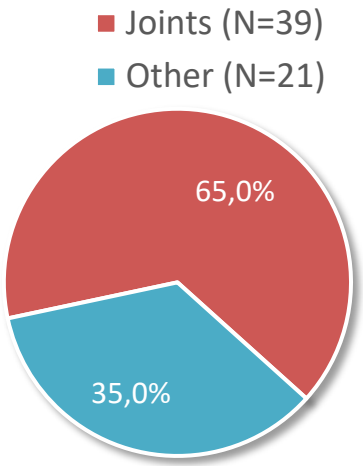
** number of bleeds*

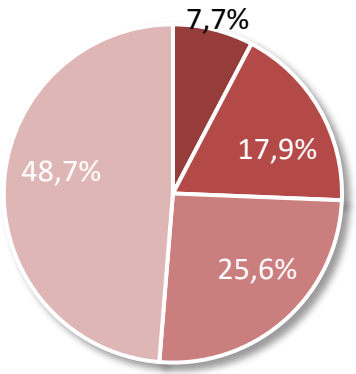
	Joints	Muscles	Subcuta-neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	*14*	*1*	*11*	*4*	*2*	*9*	*0*	*0*	*1*	***42***
FIX consumption per bleed (IU), valid N	*5*	*0*	*6*	*2*	*0*	*8*			*1*	***22***
geometric mean	6028.9		1200.9	2449.5		1090.5			12000.0	**1982.1**
median	**5000.0**		**1500.0**	**2500.0**		**1000.0**			**12000.0**	**2000.0**
min – max	3000–29500		250–4000	2000–3000		1000–2000			12000–12000	**250–29500**
sum	46 500		10 750	5 000		9 000			12 000	**83 250**
No. of doses per bleed										
geometric mean	3.2		1.3	3.1		1.0			4.0	**2.1**
median	**3**		**1**	**3**		**1**			**4**	**1**
min – max	0–16		0–3	0–5		0–1			4–4	**0–16**
Duration of therapy per bleed, days										
geometric mean	3.9		1.5	5.4		1.1			15.0	**2.6**
median	**3**		**1**	**6**		**1**			**15**	**3**
min – max	1–20		1–4	3–8		1–2			15–15	**1–20**
N (%) with hospitalization	*1 (7.1%)*		*1 (9.1%)*	*2 (50%)*	*1 (50%)*	*0 (0%)*			*0 (0%)*	***5 (11.9%)***
N (%) with rebleeding	*2 (14.3%)*	*1 (100%)*	*3 (27.3%)*	*0 (0%)*	*0 (0%)*	*1 (11.1%)*			*0 (0%)*	***7 (16.7%)***

Location and etiology of bleeds

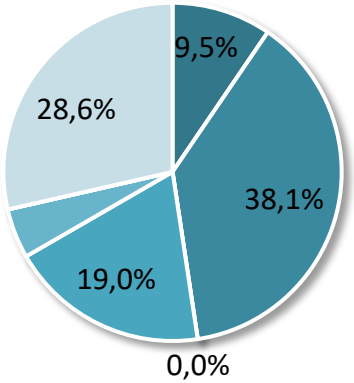
Adults
Haem B
N=60*

** number of bleeds*

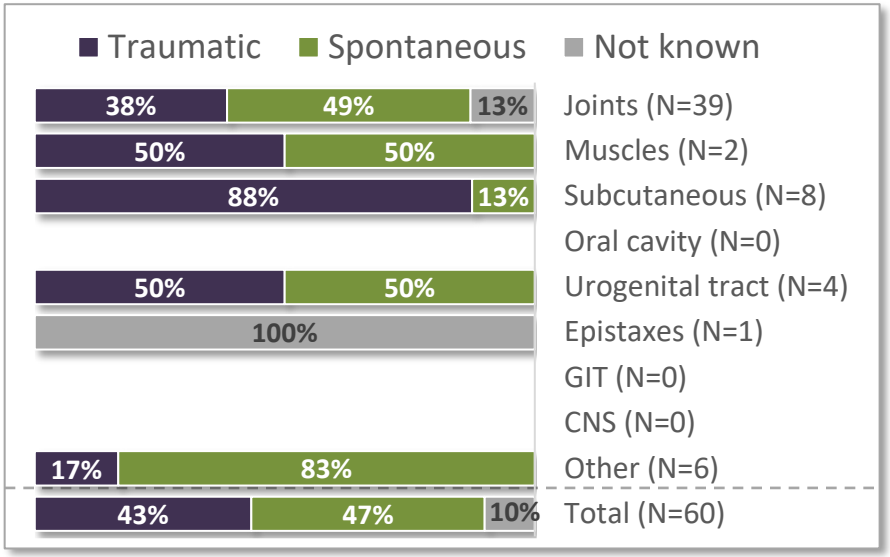




- Knee (N=3)
- Ankle (N=7)
- Elbow (N=10)
- Other joint (N=19)



- Muscles (N=2)
- Subcutaneous (N=8)
- Oral cavity (N=0)
- Urogenital tract (N=4)
- Epistaxes (N=1)
- GIT (N=0)
- CNS (N=0)
- Other (N=6)



Detailed treatment of bleeds

Adults
Haem B
N=60*

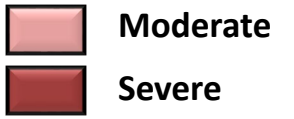
** number of bleeds*

	Joints	Muscles	Subcuta-neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	*39*	*2*	*8*	*0*	*4*	*1*	*0*	*0*	*6*	***60***
FIX consumption per bleed (IU), valid N	*38*	*2*	*7*		*4*	*1*			*6*	***58***
geometric mean	4922.4	3794.7	6097.4		8541.3	8000.0			6376.0	**5386.0**
median	**4500.0**	**4200.0**	**8000.0**		**8600.0**	**8000.0**			**4800.0**	**4800.0**
min – max	600–51000	2400–6000	1200–34000		1800–56000	8000–8000			2400–30000	**600–56000**
sum	271 000	8 400	71 200		75 000	8 000			62 400	**496 000**
No. of doses per bleed										
geometric mean	1.6	1.0	1.9		5.2	2.0			2.0	**1.8**
median	**1**	**1**	**1**		**6**	**2**			**2**	**1**
min – max	0–17	1–1	0–17		2–18	2–2			1–7	**0–18**
Duration of therapy per bleed, days										
geometric mean	1.6	1.0	1.7		3.6	1.0			2.0	**1.7**
median	**1**	**1**	**2**		**4**	**1**			**2**	**1**
min – max	1–30	1–1	1–3		1–28	1–1			1–6	**1–30**
N (%) with hospitalization	*2 (5.1%)*	*0 (0%)*	*2 (25%)*		*1 (25%)*	*0 (0%)*			*2 (33.3%)*	***7 (11.7%)***
N (%) with rebleeding	*1 (2.6%)*	*0 (0%)*	*1 (12.5%)*		*0 (0%)*	*0 (0%)*			*0 (0%)*	***2 (3.3%)***

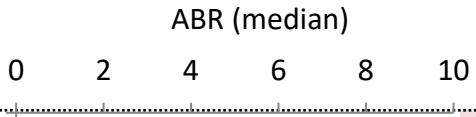
ABR according to centres Haemophilia B (PWHB)

Annual bleeding rate on permanent prophylaxis

HaemB on prophy
Paed. centres
N=10



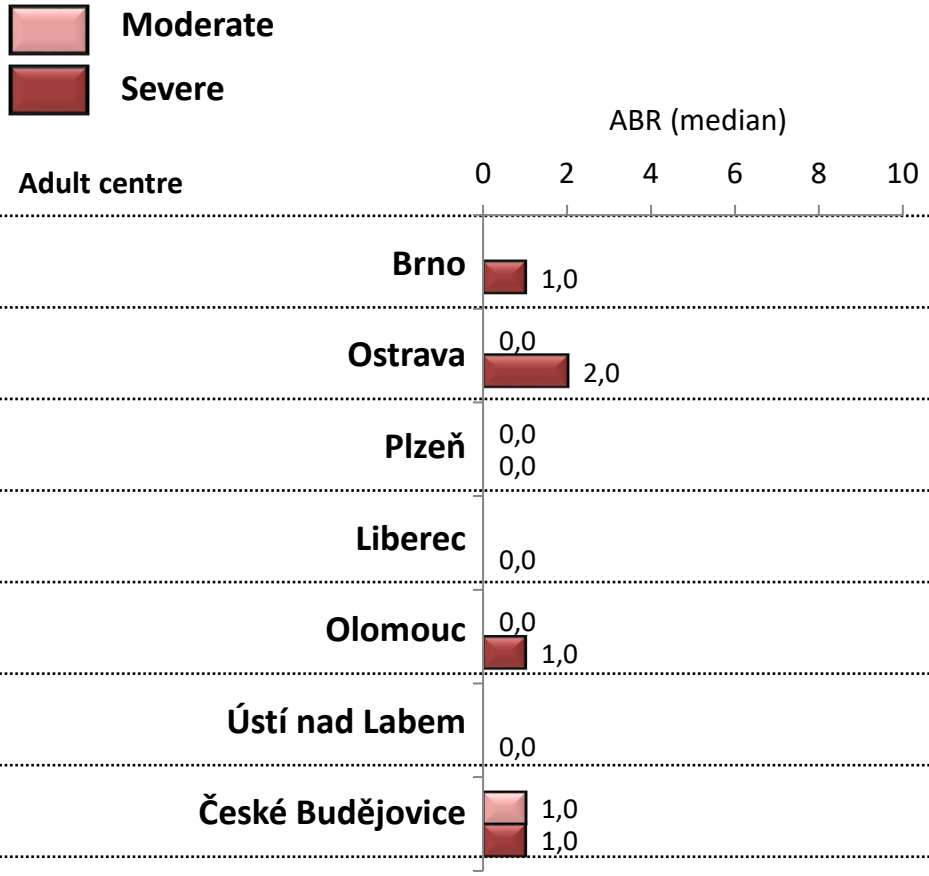
Frequency of bleeding in PWHB without inhibitor on permanent prophylaxis



Paediatric centre	N	Mean	Median	Min	Max	Severity
Praha	2	3.0	**3.0**	1	5	**Moderate**
	4	2.8	**1.0**	0	9	**Severe**
Brno	0					
	1	1.0	**1.0**	1	1	**Severe**
Ostrava	0					
	2	0.0	**0.0**	0	0	**Severe**
Ústí nad Labem	0					
	1	3.0	**3.0**	3	3	**Severe**

Annual bleeding rate on permanent prophylaxis

HaemB on prophy
Adult centres
N=17



Frequency of bleeding in PWHB without inhibitor on permanent prophylaxis

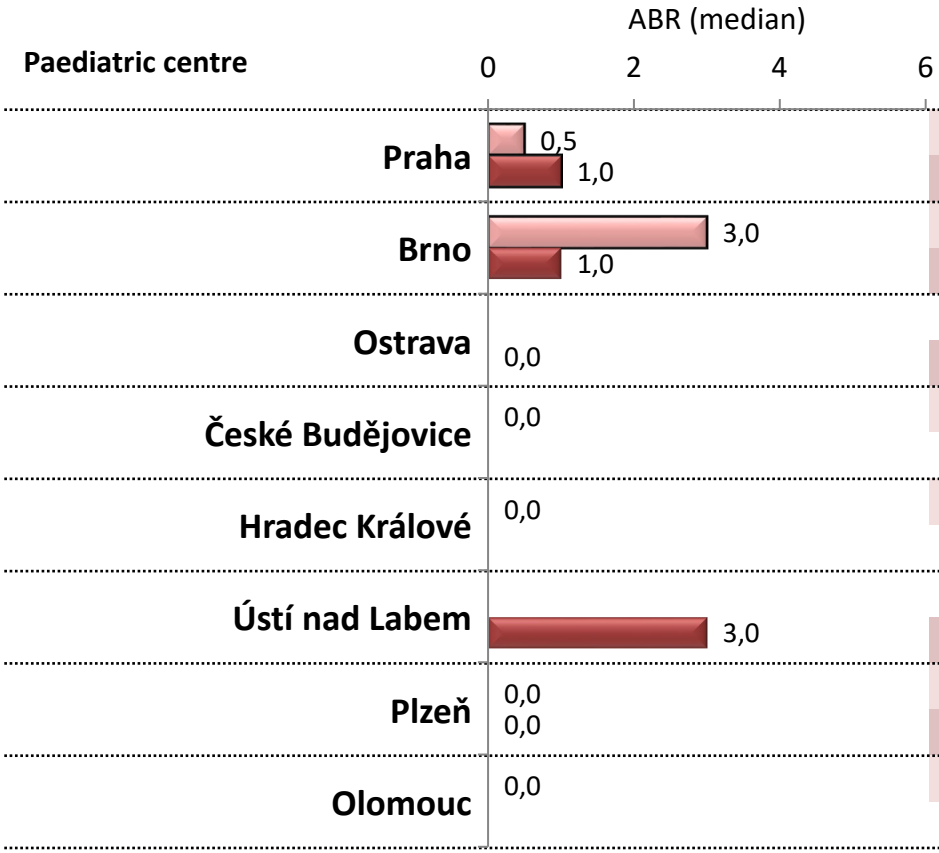
Adult centre	N	Mean	Median	Min	Max	Severity
Brno	0					
	6	1.7	**1.0**	0	6	**Severe**
Ostrava	1	0.0	**0.0**	0	0	**Moderate**
	5	4.2	**2.0**	0	11	**Severe**
Plzeň	2	0.0	**0.0**	0	0	**Moderate**
	3	2.0	**0.0**	0	6	**Severe**
Liberec	0					
	1	0.0	**0.0**	0	0	**Severe**
Olomouc	1	0.0	**0.0**	0	0	**Moderate**
	1	1.0	**1.0**	1	1	**Severe**
Ústí nad Labem	0					
	1	0.0	**0.0**	0	0	**Severe**
České Budějovice	1	1.0	**1.0**	1	1	**Moderate**
	1	1.0	**1.0**	1	1	**Severe**

Annual bleeding rate regardless prophylaxis

HaemB
Paed. centres
N=22

Moderate
Severe

Frequency of bleeding in PWHB without inhibitor regardless of prophylaxis



Paediatric centre	N	Mean	Median	Min	Max	% on permanent prophylaxis
Praha	6	1.2	**0.5**	0	5	**33.3%**
	5	2.4	**1.0**	0	9	**80.0%**
Brno	1	3.0	**3.0**	3	3	**0.0%**
	3	1.0	**1.0**	0	2	**33.3%**
Ostrava	0					
	2	0.0	**0.0**	0	0	**100.0%**
České Budějovice	0	0.0	**0.0**	0	0	**0.0%**
	0					
Hradec Králové	1	0.0	**0.0**	0	0	**0.0%**
	0					
Ústí nad Labem	0					
	1	3.0	**3.0**	3	3	**100.0%**
Plzeň	1	0.0	**0.0**	0	0	**0.0%**
	1	0.0	**0.0**	0	0	**100.0%**
Olomouc	1	0.0	**0.0**	0	0	**0.0%**
	0					

Annual bleeding rate regardless prophylaxis

HaemB
Adult centres
N=48*

** missing ABR in 1 adult*

Moderate
Severe

Frequency of bleeding in PWHB without inhibitor regardless of prophylaxis

Adult centre	N*	Mean	Median	Min	Max	% on permanent prophylaxis
Brno	5	0.2	**0.0**	0	1	**0.0%**
	7	2.1	**1.0**	0	6	**85.7%**
Ostrava	3	0.0	**0.0**	0	0	**33.3%**
	7	3.0	**2.0**	0	11	**71.4%**
Plzeň	3	0.0	**0.0**	0	0	**66.7%**
	4	1.5	**0.0**	0	6	**75.0%**
Liberec	2	0.0	**0.0**	0	0	**0.0%**
	1	0.0	**0.0**	0	0	**100.0%**
Olomouc	8	1.1	**0.0**	0	6	**12.5%**
	2	0.5	**0.5**	0	1	**33.3%**
Ústí nad Labem	0					
	2	1.5	**1.5**	0	3	**50.0%**
České Budějovice	3	0.3	**0.0**	0	1	**33.3%**
	1	1.0	**1.0**	1	1	**100.0%**

Prophylactic regimens and treatment outcomes

HaemB
Paed. centres
N=22

Paediatric centre	Severity	Total N	PERMANENT PROPHYLAXIS								ON-DEMAND / TEMPORARY PROPHY		
			% of patients	N	Dosing of **SHL** prophylaxis (IU/kg per week)		Dosing of **EHL** prophylaxis (IU/kg per week)		ABR		N	ABR	
					Mean	Median	Mean	Median	Mean	Median		Mean	Median
Praha	**Moderate**	6	**33.3%**	2			30.8	**30.8**	3.0	**3.0**	4	0.3	**0.0**
	Severe	5	**80.0%**	4			48.9	**50.8**	2.8	**1.0**	1	1.0	**1.0**
Brno	**Moderate**	1	**0.0%**	0							1	3.0	**3.0**
	Severe	3	**33.3%**	1			34.5	**34.5**	1.0	**1.0**	2	1.0	**1.0**
Ostrava	**Moderate**	0	**0.0%**	0							0		
	Severe	2	**100.0%**	2	58.9	**58.9**	17.3	**17.3**	0.0	**0.0**	0		
Č. Budějovice	**Moderate**	0	**0.0%**	0							0		
	Severe	0	**0.0%**	0							0		
Hradec Králové	**Moderate**	1	**0.0%**	0							1	0.0	**0.0**
	Severe	0	**0.0%**	0							0		
Ústí nad Labem	**Moderate**	0	**0.0%**	0							0		
	Severe	1	**100.0%**	1			39.0	**39.0**	3.0	**3.0**	0		
Plzeň	**Moderate**	1	**0.0%**	0							1	0.0	**0.0**
	Severe	1	**100.0%**	1			52.4	**52.4**	0.0	**0.0**	0		
Olomouc	**Moderate**	1	**0.0%**	0							1	0.0	**0.0**
	Severe	0	**0.0%**	0							0		

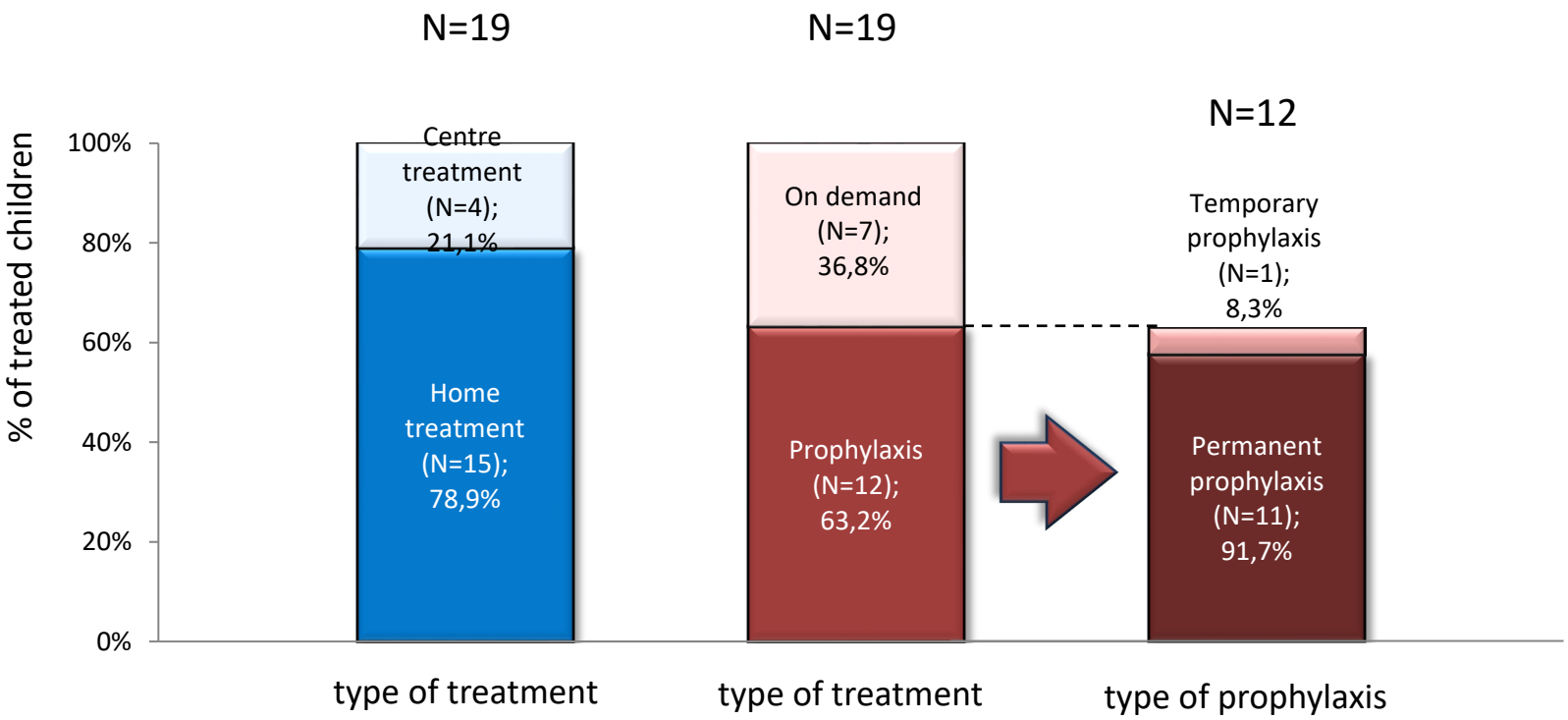
Prophylactic regimens and treatment outcomes

HaemB
Adult centres
N=49

Adult centre	Severity	Total N	PERMANENT PROPHYLAXIS									ON-DEMAND / TEMPORARY PROPHY			
			% of patients	N	Dosing of **SHL** prophylaxis (IU/kg per week)		Dosing of **EHL** prophylaxis (IU/kg per week)		ABR		Age	N	ABR		Age
					Mean	Median	Mean	Median	Mean	Median	Median		Mean	Median	Median
Brno	**Moderate**	5	**0.0%**	0								5	0.2	**0.0**	51
	Severe	7	**85.7%**	6			36.4	**34.4**	1.7	**1.0**	34	1	5.0	**5.0**	21
Ostrava	**Moderate**	3	**33.3%**	1			27.6	**27.6**	0.0	**0.0**	24	2	0.0	**0.0**	62
	Severe	7	**71.4%**	5	64.4	**64.4**	54.2	**53.4**	4.2	**2.0**	56	2	0.0	**0.0**	43
Plzeň	**Moderate**	3	**66.7%**	2			22.7	22.7	0.0	**0.0**	57	1	0.0	**0.0**	61
	Severe	4	**75.0%**	3			18.8	**18.2**	2.0	**0.0**	47	1	0.0	**0.0**	35
Liberec	**Moderate**	2	**0.0%**	0								2	0.0	**0.0**	35
	Severe	1	**100.0%**	1			71.6	**71.6**	0.0	**0.0**	31	0			
Olomouc	**Moderate**	8	**12.5%**	1			20.2	20.2	0.0	**0.0**	44	7	1.3	**0.0**	55
	Severe	3	**33.3%**	1			18.5	**18.5**	1.0	**1.0**	54	2	0.0	**0.0**	44
Ústí n. Labem	**Moderate**	0	**0.0%**	0								0			
	Severe	2	**50.0%**	1			19.2	**19.2**	0.0	**0.0**	28	1	3.0	**3.0**	52
Č. Budějovice	**Moderate**	3	**33.3%**	1			83.6	83.6	1.0	**1.0**	57	2	0.0	**0.0**	48
	Severe	1	**100.0%**	1			17.9	**17.9**	1.0	**1.0**	50	0			

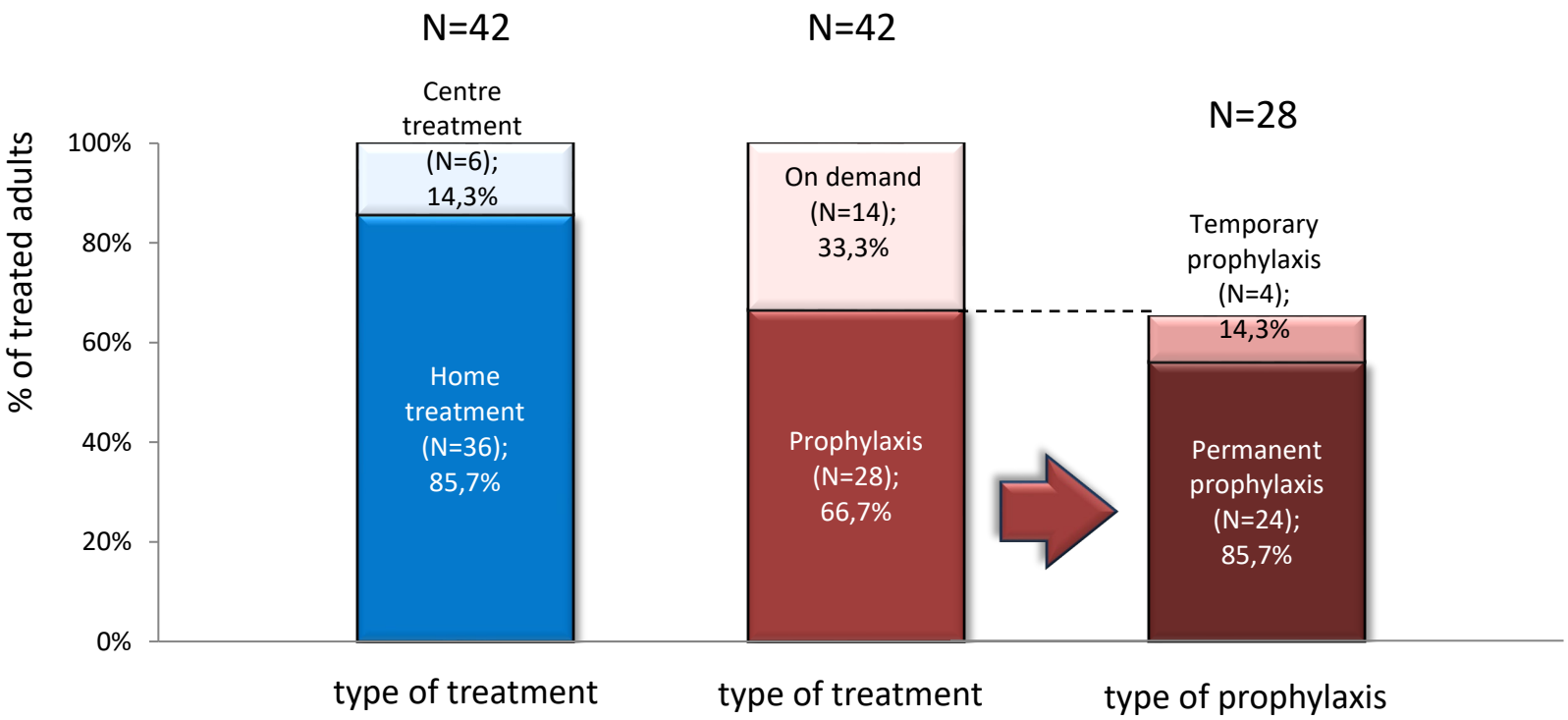
Children
Haem B
N=19

Type of treatment (subgroup of treated patients)



Adults
Haem B
N=42

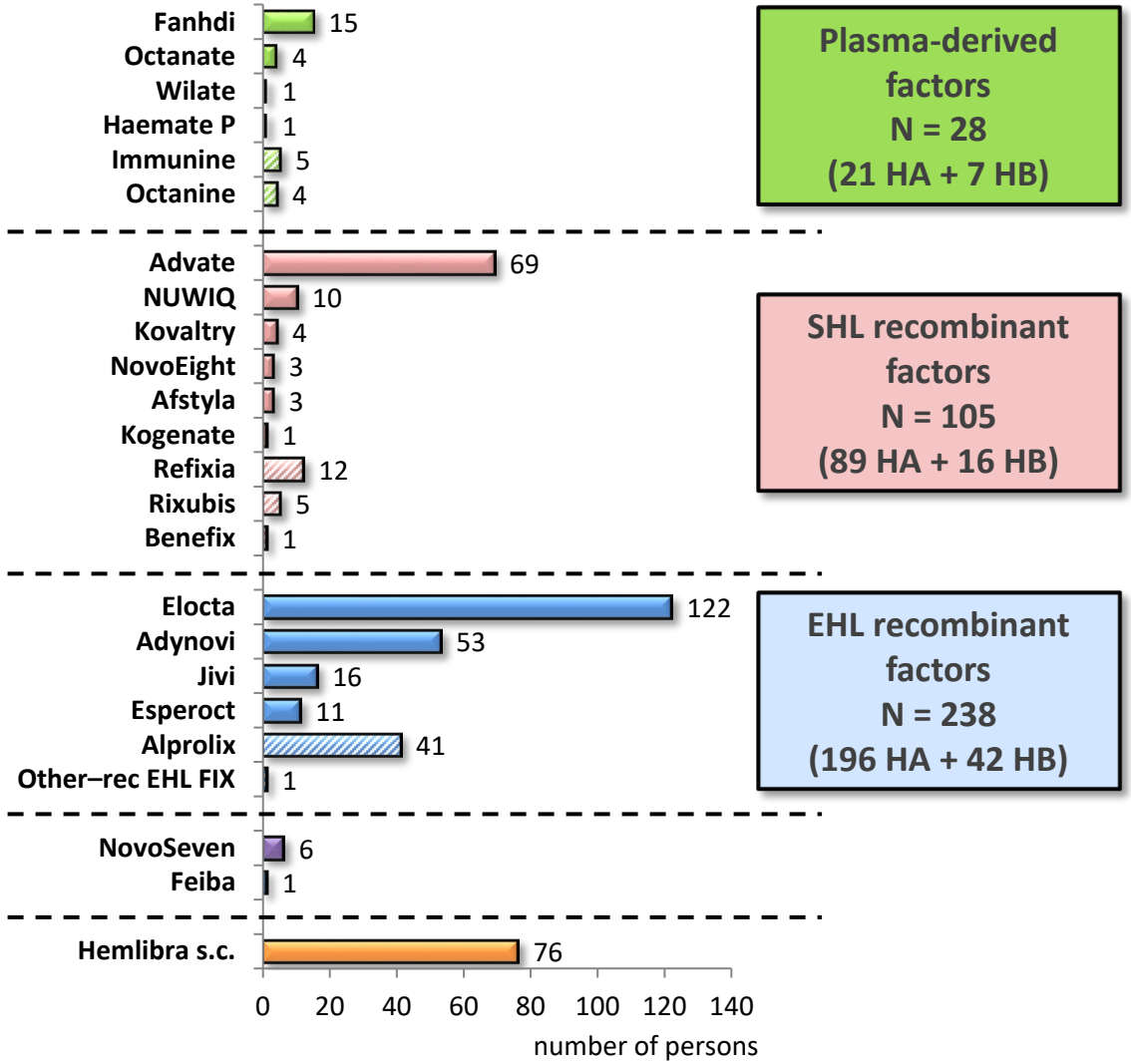
Type of treatment (subgroup of treated patients)



Treatment data and factor consumption Haemophilia A and B

Treatment

All
N=393[1]

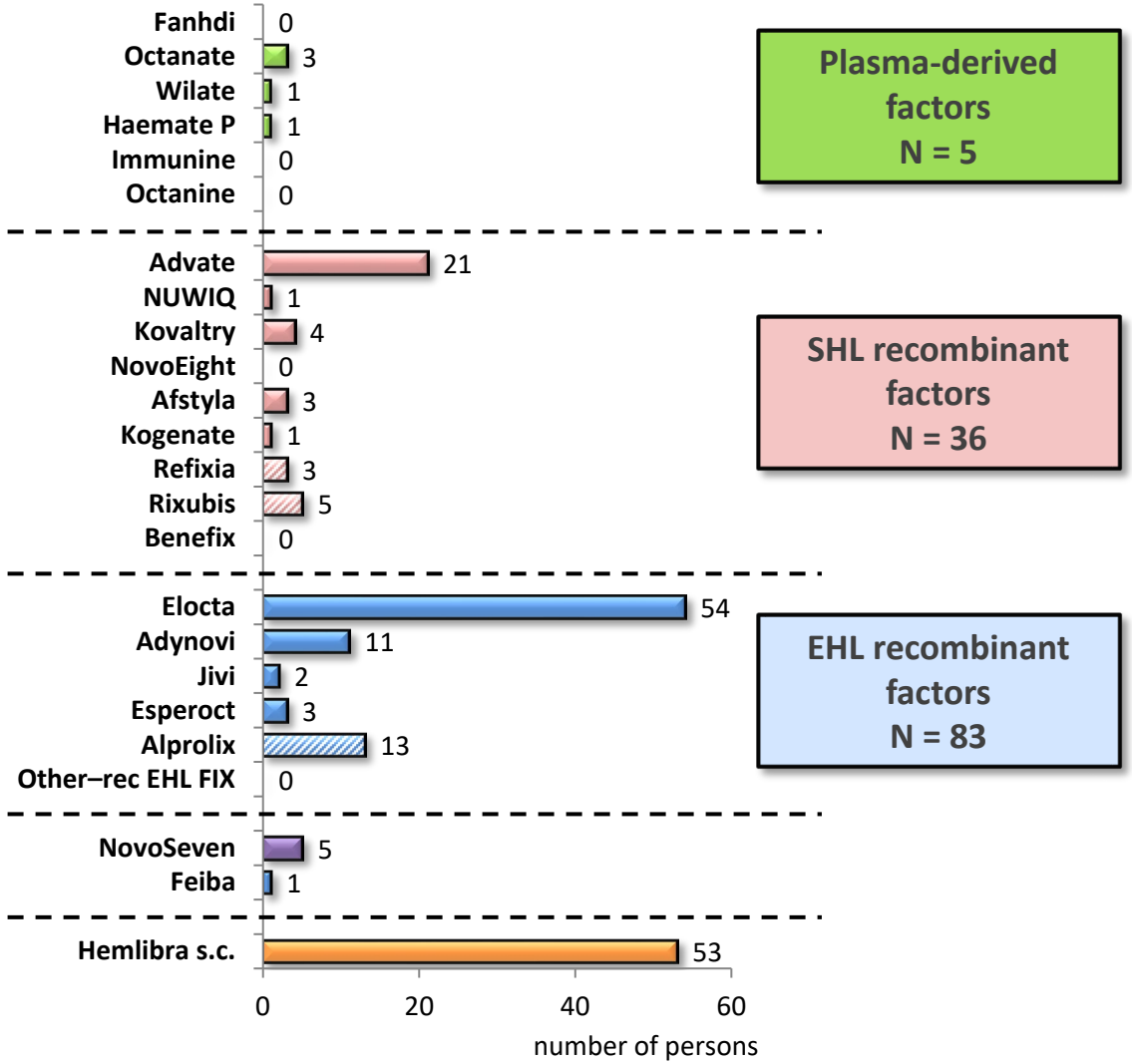


395 persons (53% of all PWH) were treated in 2023 (121 persons received standard factor concentrates, 238 persons received EHL factors, 6 by-pass therapy and 76 emicizumab, in 2 persons data are not available; 61 persons received more than one type/brand of concentrate).
12 persons were treated with both plasma-derived and recom-binant factor.

[1]*missing type of treatment in 2 adults*

Treatment

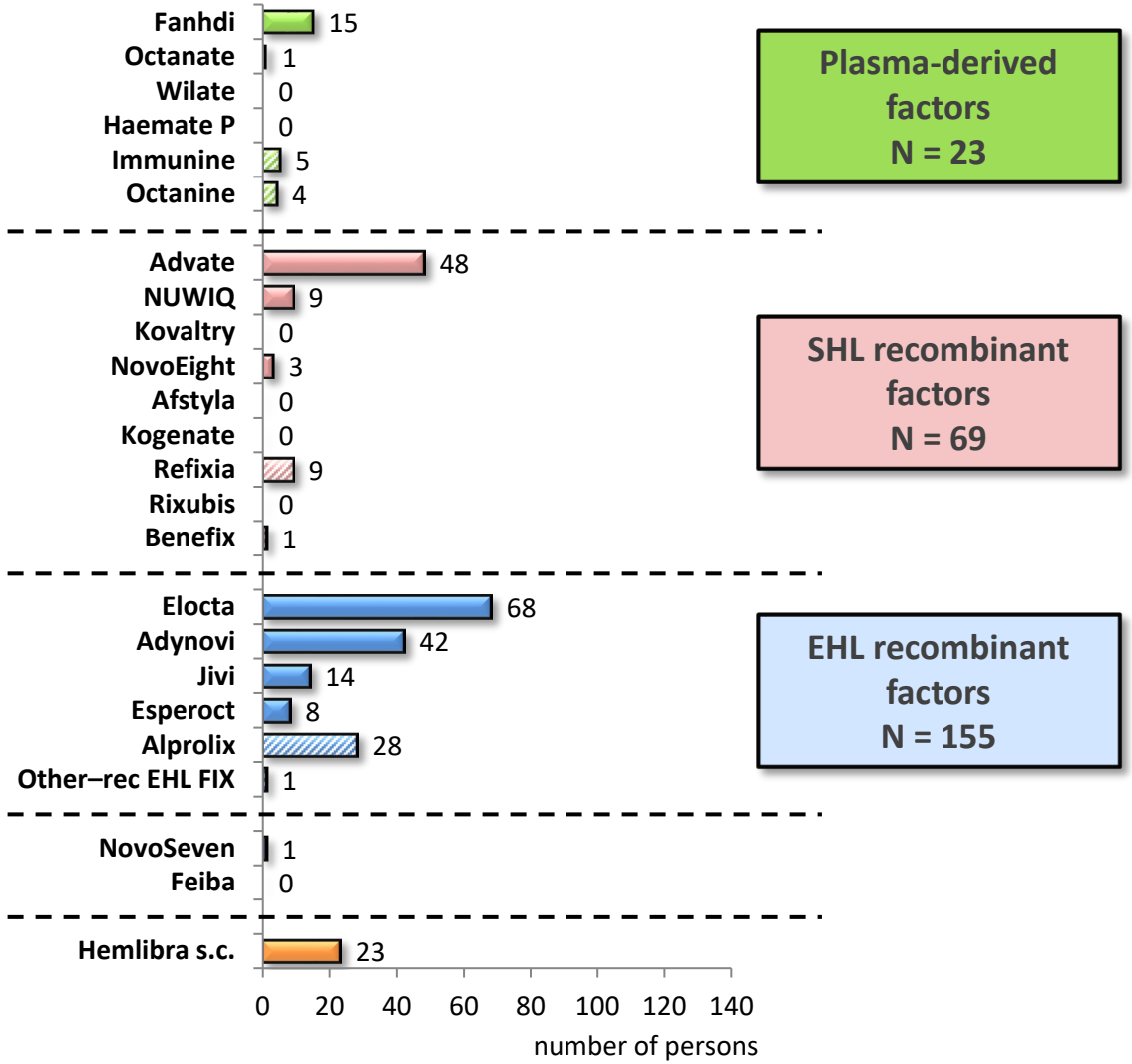
Children
N=154



154 children (54.2% of all PWH) were treated in 2023 (38 children received standard factor concentrates, 83 EHL factors, 5 by-pass therapy and 53 children emicizumab; 29 children received more than one type/brand of concentrate). Three children were treated with both plasma-derived and recombinant factor.

Treatment

Adults
N=239[1]



241 adults (52.3% of all PWH) were treated in 2023 (83 adults received standard factor concentrates, 155 EHL factors, 1 by-pass therapy and 23 adults emicizumab; 32 adults received more than one type/brand of concentrate).
9 adults were treated with both plasma-derived and recombinant factor.

[1]*missing type of treatment in 2 adults*

All

Comparison of treatment in years 2023 and 2022

	2023			2022		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All treated persons *	395	53.0	100.0	394	52.0	100.0
Plasma-derived factor	28	*3.8*	*7.1*	*49*	*6.5*	*12.4*
Recombinant factor	105	*14.1*	*26.6*	*126*	*16.6*	*32.0*
Recombinant f. EHL	238	*31.9*	*60.3*	*206*	*27.2*	*52.3*
Emicizumab	76	*10.2*	*19.2*	*62*	*8.2*	*15.7*
Without treatment	350	47.0	-	363	48.0	-
Total	745	100.0	-	757	100.0	-

** One patient could have more type of factor concentrates and/or emicizumab.*

Children

Comparison of treatment in years 2023 and 2022

	2023			2022		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All treated persons *	154	54.2	100.0	152	53.1	100.0
Plasma-derived factor	*5*	*1.8*	*3.2*	*6*	*2.1*	*3.9*
Recombinant factor	*36*	*12.7*	*23.4*	*49*	*17.1*	*32.2*
Recombinant f. EHL	*83*	*29.2*	*53.9*	*78*	*27.3*	*51.3*
Emicizumab	*53*	*18.7*	*34.4*	*48*	*16.8*	*31.6*
Without treatment	130	45.8	-	134	46.9	-
Total	284	100.0	-	286	100.0	-

** One patient could have more type of factor concentrates and/or emicizumab.*

Adults

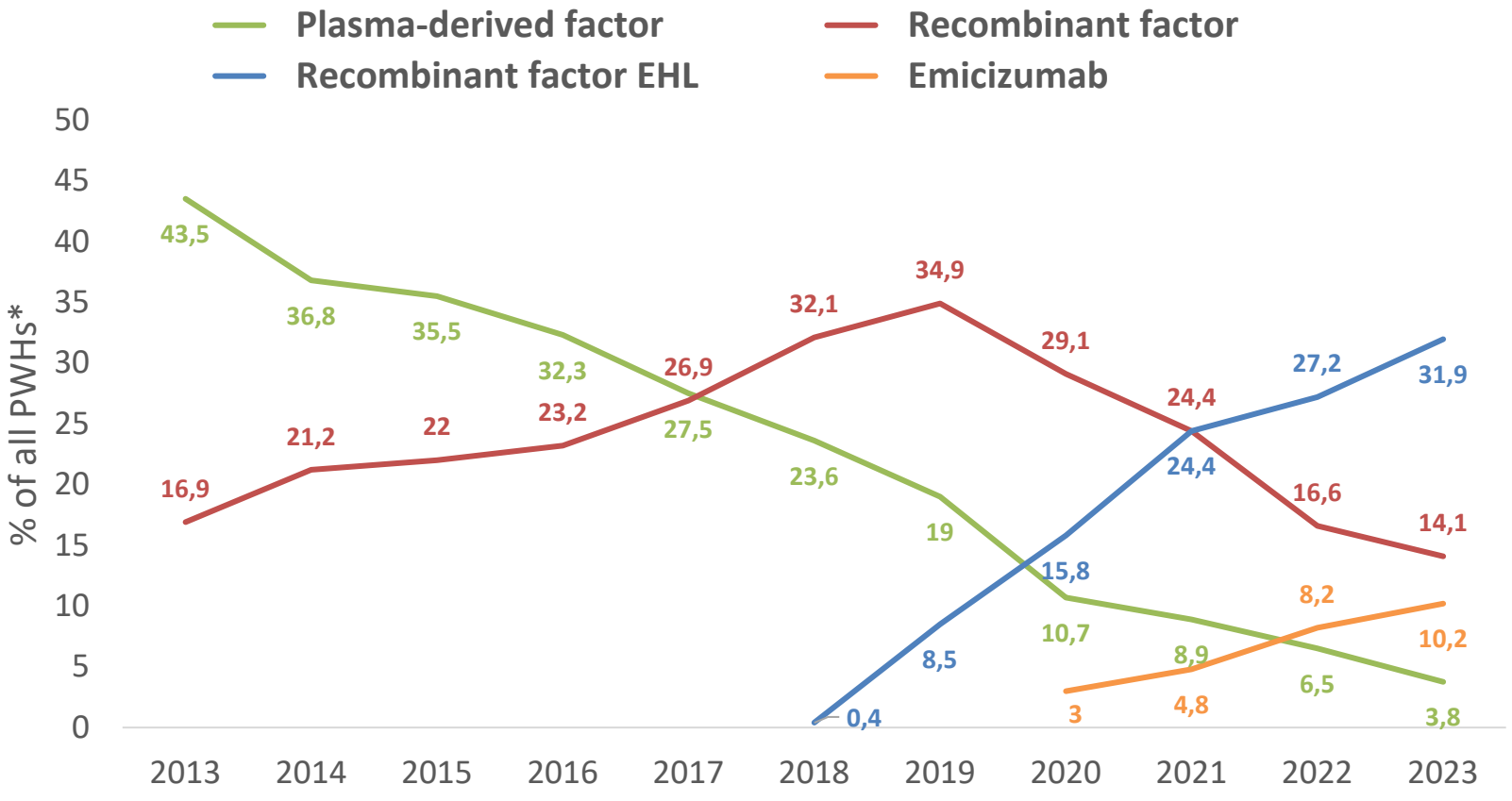
Comparison of treatment in years 2023 and 2022

	2023			2022		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All treated persons *	241	52.3	100.0	242	51.4	100.0
Plasma-derived factor	23	*5.0*	*9.5*	*43*	*9.1*	*17.8*
Recombinant factor	69	*15.0*	*28.6*	*77*	*16.3*	*31.8*
Recombinant f. EHL	155	*33.6*	*64.3*	*128*	*27.2*	*52.9*
Emicizumab	23	*5.0*	*9.5*	*14*	*3.0*	*5.8*
Without treatment	220	47.7	-	229	48.6	-
Total	461	100.0	-	471	100.0	-

** One patient could have more type of factor concentrates and/or emicizumab.*

Comparison of treatment in years

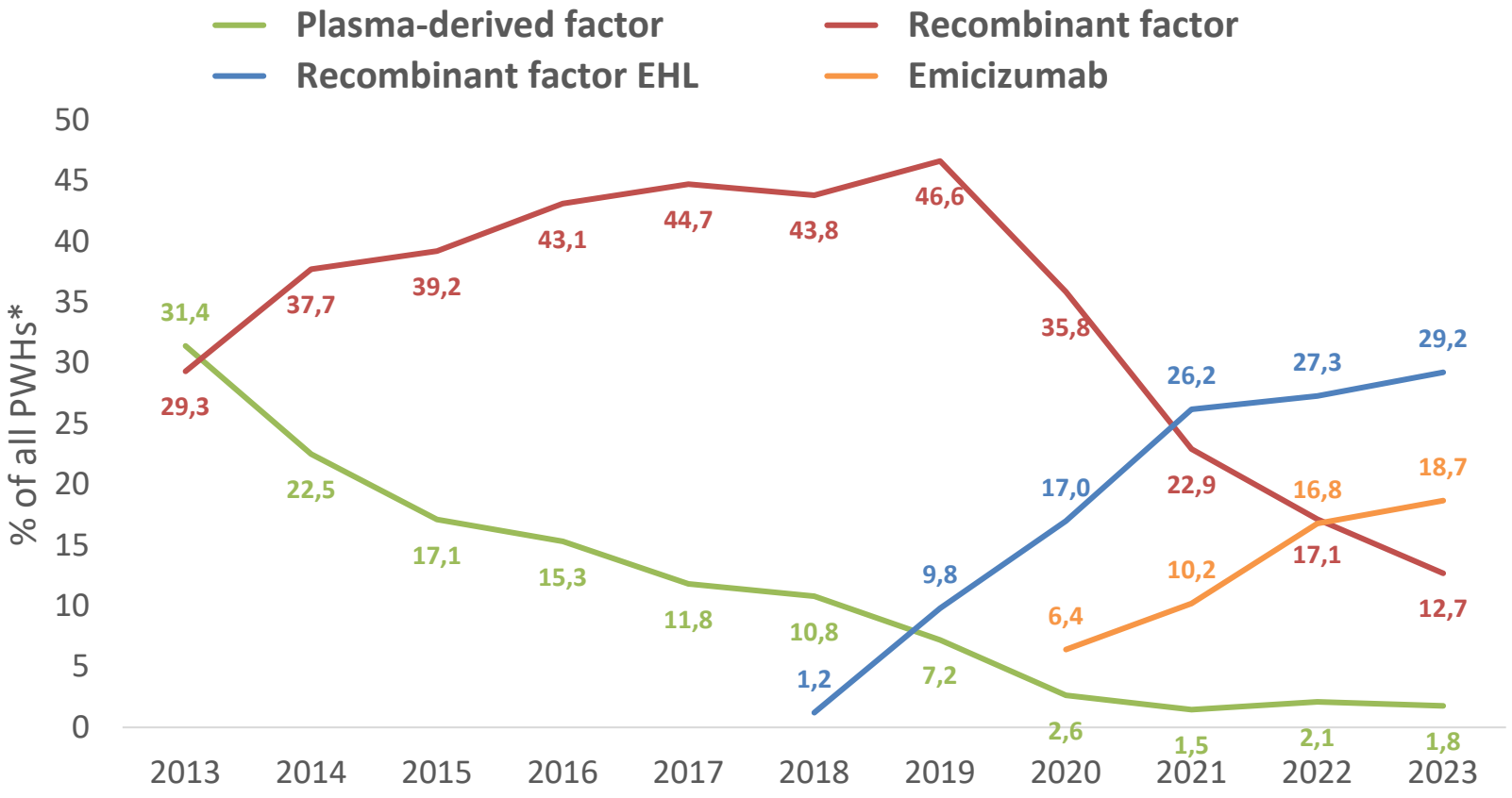
All



** One patient could have more type of treatments*

Comparison of treatment in years

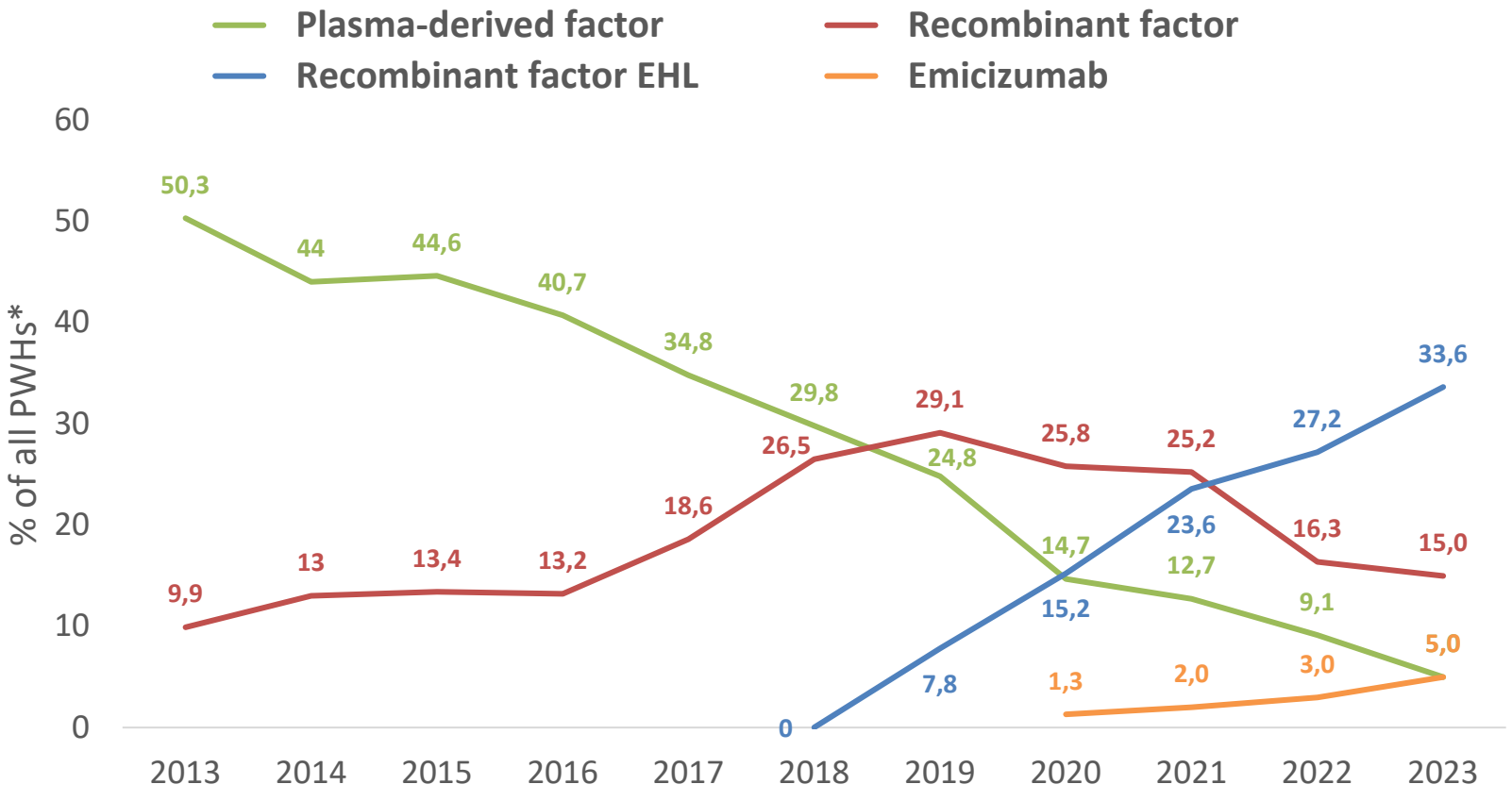
Children



** One patient could have more type of treatments*

Comparison of treatment in years

Adults



** One patient could have more type of treatments*

Consumption of drugs

All

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	Fanhdi	1 123 550	15	74 903.3
	Octanate	761 000	4	190 250.0
	Wilate	2 500	1	2 500.0
	Haemate P	2 400	1	2 400.0
	PD FVIII total	**1 889 450**	**21**	**89 973.8**
	Advate	3 055 510	69	44 282.8
	NUWIQ	1 952 125	10	195 212.5
	Kovaltry	25 000	4	6 250.0
	NovoEight	403 000	3	134 333.3
	Afstyla	500 000	3	166 666.7
	Kogenate	1 000	1	1 000.0
	SHL REC FVIII total	**5 936 635**	**89**	**66 703.8**
	Standard FVIII total	*7 826 085*	*110*	*71 146.2*
	Elocta	18 372 270	121	151 836.9
	Adynovi	11 469 045	53	216 397.1
	Jivi	3 667 770	16	229 235.6
	Esperoct	2 600 720	11	236 429.1
	EHL REC FVIII total	*36 109 805*	*196*	*184 233.7*
	FVIII total	**43 935 890**	**286**	**153 622.0**
FIX (IU)	Immunine	230 400	5	46 080.0
	Octanine	255 000	4	63 750.0
	FIX PD total	**485 400**	**7**	**69 342.9**
	Refixia	839 180	12	69 931.7
	Rixubis	160 000	5	32 000.0
	Benefix	30 000	1	30 000.0
	FIX REC total	**1 029 180**	**16**	**64 323.8**
	Standard FIX total	*1 514 580*	*21*	*72 122.9*
	Alprolix	4 098 974	41	99 975.0
	Other–rec EHL FIX	84 000	1	84 000.0
	EHL REC FIX total	*4 182 974*	*42*	*99 594.6*
	FIX total	**5 697 554**	**59**	**96 568.7**
By-pass	NovoSeven (mg)	1 453.6	6	242.3
Emicizumab	Hemlibra s.c. (mg)	229 952	76	3 025.7

Consumption of drugs

Children

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	Fanhdi	0	0	
	Octanate	751 000	3	250 333.3
	Wilate	2 500	1	2 500.0
	Haemate P	2 400	1	2 400.0
	PD FVIII total	755 900	5	151 180.0
	Advate	575 750	21	27 416.7
	NUWIQ	2 000	1	2 000.0
	Kovaltry	25 000	4	6 250.0
	NovoEight	0	0	
	Afstyla	500 000	3	166 666.7
	Kogenate	1 000	1	1 000.0
	SHL REC FVIII total	1 103 750	30	36 791.7
	Standard FVIII total	1 859 650	35	53 132.9
	Elocta	5 617 550	54	104 028.7
	Adynovi	2 149 000	11	195 363.6
	Jivi	670 000	2	335 000.0
	Esperoct	472 000	3	157 333.3
	EHL REC FVIII total	8 908 550	70	127 265.0
	FVIII total	10 768 200	98	109 879.6
FIX (IU)	Immunine	0	0	
	Octanine	0	0	
	FIX PD total	0	0	
	Refixia	81 500	3	27 166.7
	Rixubis	160 000	5	32 000.0
	Benefix	0	0	
	FIX REC total	241 500	6	40 250.0
	Standard FIX total	241 500	6	40 250.0
	Alprolix	800 350	13	61 565.4
	Other–rec EHL FIX	0	0	
	EHL REC FIX total	800 350	13	61 565.4
	FIX total	1 041 850	18	57 880.6
By-pass	NovoSeven (mg)	1 438.6	5	287.7
Emicizumab	Hemlibra s.c. (mg)	89 836	53	1 695.0

Consumption of drugs

Adults

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	Fanhdi	1 123 550	15	74 903.3
	Octanate	10 000	1	10 000.0
	Wilate	0	0	
	Haemate P	0	0	
	PD FVIII total	1 133 550	16	70 846.9
	Advate	2 479 760	48	51 661.7
	NUWIQ	1 950 125	9	216 680.6
	Kovaltry	0	0	
	NovoEight	403 000	3	134 333.3
	Afstyla	0	0	
	Kogenate	0	0	
	SHL REC FVIII total	4 832 885	59	81 913.3
	Standard FVIII total	5 966 435	75	79 552.5
	Elocta	12 754 720	67	190 369.0
	Adynovi	9 320 045	42	221 905.8
	Jivi	2 997 770	14	214 126.4
	Esperoct	2 128 720	8	266 090.0
	EHL REC FVIII total	27 201 255	126	215 883.0
	FVIII total	33 167 690	188	176 423.9
FIX (IU)	Immunine	230 400	5	46 080.0
	Octanine	255 000	4	63 750.0
	FIX PD total	485 400	7	69 342.9
	Refixia	757 680	9	84 186.7
	Rixubis	0	0	
	Benefix	30 000	1	30 000.0
	FIX REC total	787 680	10	78 768.0
	Standard FIX total	1 273 080	15	84 872.0
	Alprolix	3 298 624	28	117 808.0
	Other–rec EHL FIX	84 000	1	84 000.0
	EHL REC FIX total	3 382 624	29	116 642.2
	FIX total	4 655 704	41	113 553.8
By-pass	NovoSeven (mg)	15.0	1	15.0
Emicizumab	Hemlibra s.c. (mg)	140 117	23	6 092.0