

JEDNÁNÍ KR A VR ČNHP

1.11.2022, Brno

Brněnské hematologické dny

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Program

17:00 Zahájení

1. Zpráva o výsledcích léčby hemofilie v ČR za rok 2021 – data ČNHP (Blatný)
2. Zpráva o auditech center ČNHP (Zápotocká, Blatný)
3. Informace z PedNet (Blatný, Zápotocká)
4. Změny v registru, otázky k datamanagementu (Ovesná)
5. Plán data-monitoringu na rok 2023 (Zápotocká, Blatný, Čermáková)
6. Zpráva o rozpočtu (výdaje/náklady) CNHP za rok 2022 (Blatný)
7. Domluva na standardizaci cen za analýzy požadované po ČNHP komerčními subjekty (Blatný, Křístková)
8. Aktualizace deklarace CNHP (Smejkal, Zápotocká) – původní text je na webu ČNHP
9. Integrace dětských dat o ITP do registru CNHP ve spolupráci s PSDH – Pracovní skupina pro dětskou hematologii (Blatný)
10. Návrh změny grafiky webu ČNHP (Blatný)
11. Návrhy na akademické analýzy/PhD projekty nad daty ČNHP (Zápotocká, všichni)
12. Různé a diskuse

19:00 Závěr:



Zpráva o výsledcích léčby hemofilie v ČR za rok 2021 – data ČNHP



The status of care for persons with haemophilia registered within CNHP registry Annual Report 2021

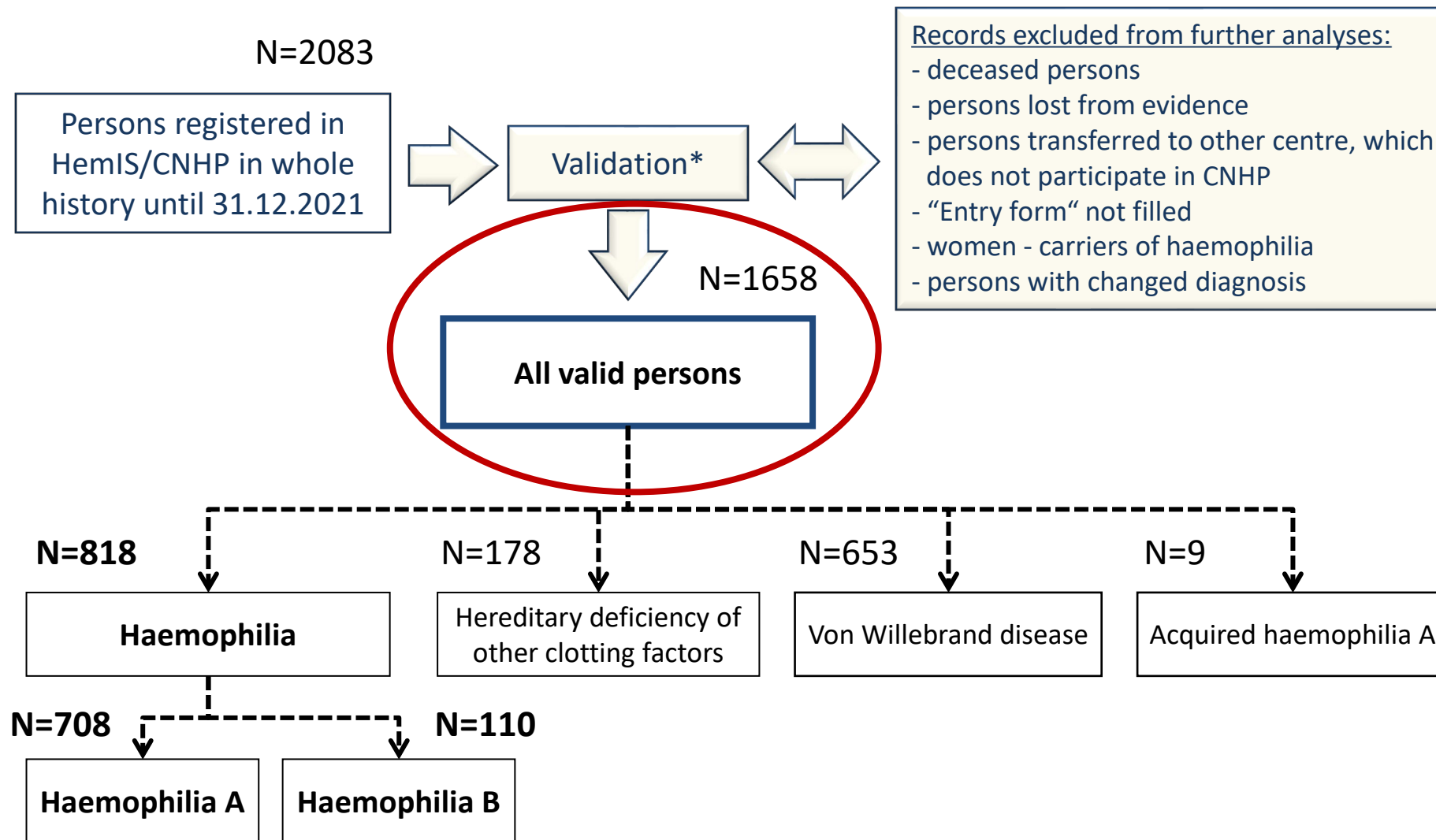
Jan Blatný, Petra Ovesná

on behalf of

Centres contributing to CNHP registry
(Czech National Haemophilia Programme)

Export date: April 01, 2022

Sample size, valid records



Persons with haemophilia and inhibitors in 2021

Active inhibitors were recorded in 19 persons in the end of year 2021

- 2 inhibitors in children with severe HA and 1 inhibitor in adult with mild HA newly developed in 2021

PWH with inhibitors:

- 11 children and 8 adults
- 18 haemophilia A and 1 haemophilia B
- 15 in severe, 2 in moderate and 2 in mild haemophilia
- 15 high-titre and 4 low-titre (<5BU)
- 9 high response and 8 low response inhibitors; this information not available in 2 PWH with inhibitors
- **15 patients were treated with emicizumab**
 - 9 patients were treated only with emi, 5 patients with emi and rFVIIa, and 1 patient with emi and rFVIII during the year
- 1 patients was treated only with rFVIIa
- 2 patients were without any „by-pass“ therapy or emi therapy and 1 patient was without any recorded treatment at all




ITT:

- One patient has already been on-going ITT in 2021 (started earlier).
- One patient started ITT in 2021.
- One patient finished ITT unsuccessfully during 2020.




ABR and treatment regimens in patients with inhibitor

N=19

	Type	Year of birth	Severity	ITT	Emi prophylaxis	By-pass prophylaxis	Titre	Responder	ABR	Joint / other
1	HA	2020	Severe	Yes	Permanent	OD	High (>5 BU/ml)	LR	2	0 / 2
2	HA	2019	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
3	HA	2018	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
4	HA	2018	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
5	HA	2017	Severe	Yes	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
6	HA	2016	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	NA	1	0 / 1
7	HA	2016	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
8	HA	2015	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
9	HA	2011	Moderate	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
10	HA	2004	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
11	HA	1977	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	1	1 / 0
12	HA	1975	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
13	HA	1971	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
14	HA	1971	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	2	2 / 0
15	HA	1956	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
16	HA	1949	Mild	No/NA	Permanent	OD	High (>5 BU/ml)	NA	0	0 / 0
17	HA	1941	Mild	Yes	Permanent	OD	High (>5 BU/ml)	LR	1	1 / 0
18	HA	1941	Moderate	No/NA	Permanent	OD	High (>5 BU/ml)	LR	0	0 / 0
19	HB	2007	Severe	No/NA	Permanent	OD	High (>5 BU/ml)	LR	17	12 / 5

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 2021

NA – not available

ABR according to treatment regimen in PWH with inhibitor

N=19

Diagnosis	ITT	Emi/by-pass prophylaxis	N	ABR (mean)	ABR (median, min-max)	Joint / other bleeds (median)
Haemophilia A	Yes	Emi permanent	1	0.00	0 (0-0)	0 / 0
		OD	1	1.00	1 (1-1)	1 / 0
	No	Emi permanent	14	0.29	0 (0-2)	0 / 0
		OD	2	1.00	1 (0-2)	2 / 0
Haemophilia B	No	BPA permanent	1	17.00	17 (17-17)	12 / 5

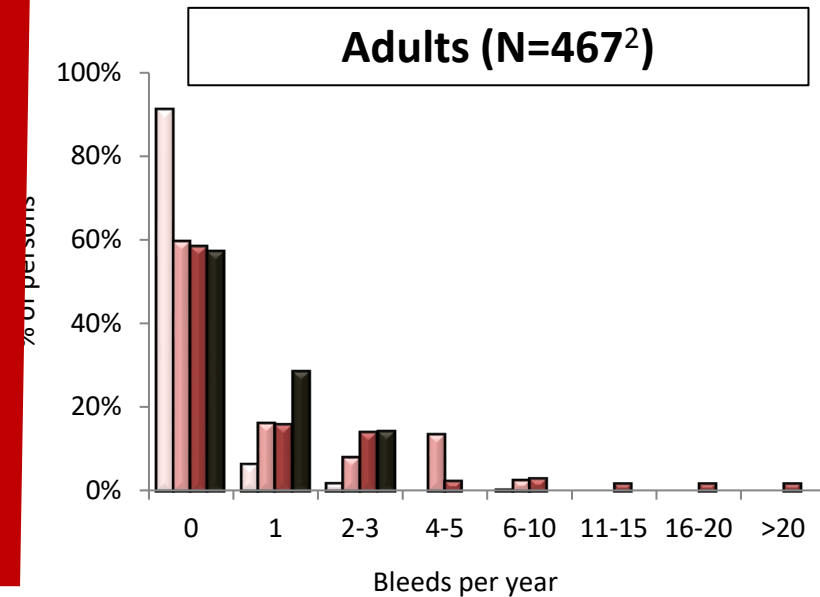
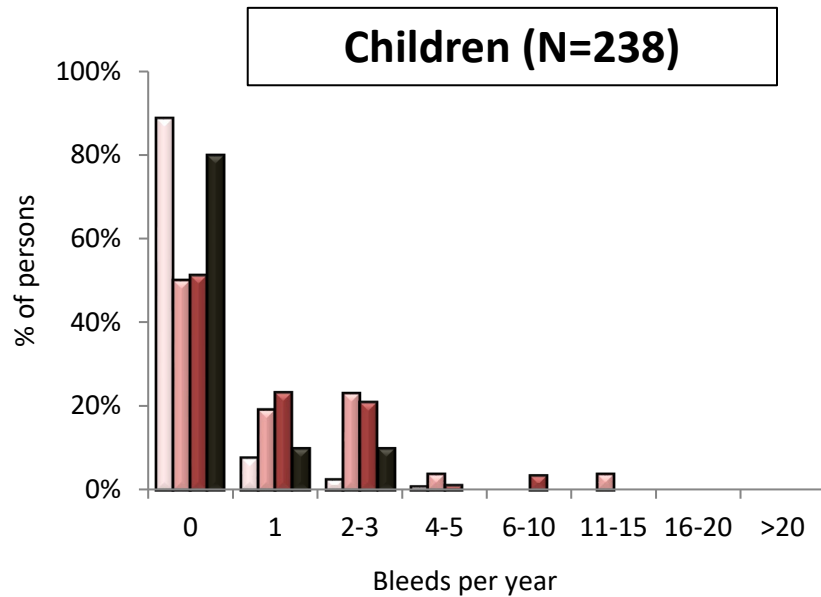
Treatment outcomes and bleeding frequency

Haemophilia A



Frequency of bleeding requiring treatment 2021

¹ severity of haemophilia not known in 2 adults



Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
116	26	86	10		261	37	162	7
0.2	1.5	1.0	0.3		0.1	1.2	1.8	0.6
0 (0-4)	0.5 (0-13)	0 (0-7)	0 (0-2)	M (0-max)	0 (0-7)	0 (0-7)	0 (0-24)	0 (0-2)
103 (88.8%)	13 (50%)	44 (51.2%)	8 (80%)	N (%) with no bleed	238 (91.2%)	22 (59.5%)	95 (58.3%)	4 (57.1%)

* without inhibitor

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

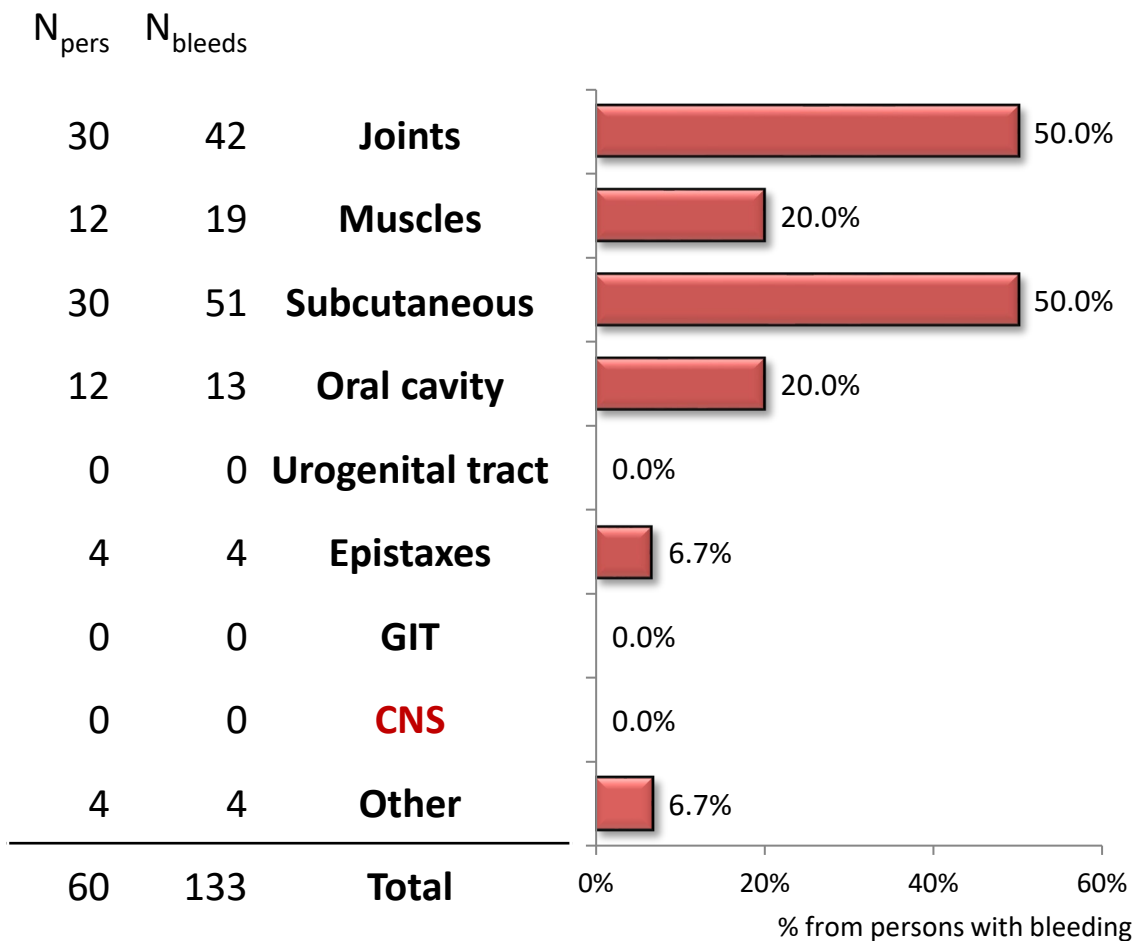
Location of bleeds in 2021

Children
Haem A
N=238

70 (29.4%) children experienced bleeding requiring treatment at least once in year; 133 bleeds were recorded in total, 12 bleeds required hospitalization.

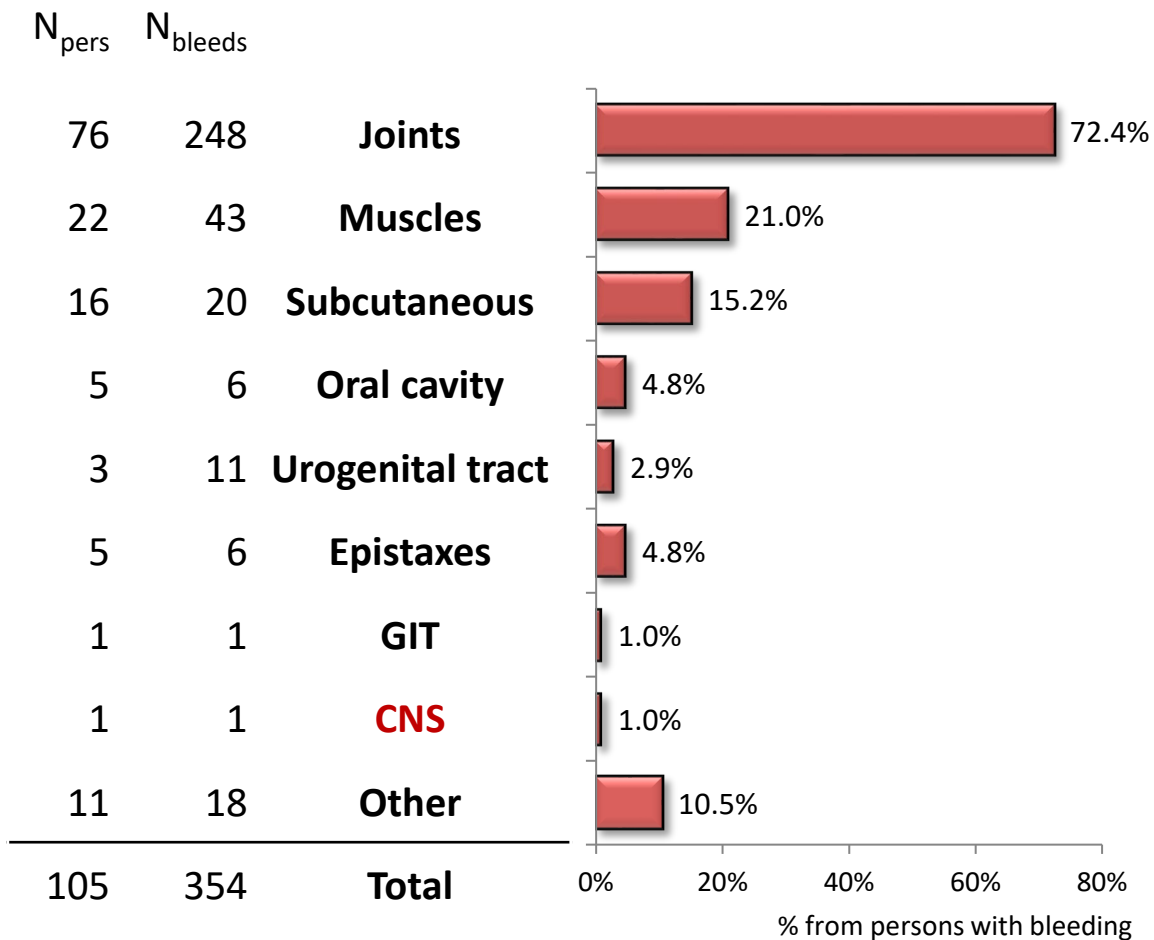
60 of these 70 children have recorded location of their bleeds. Localization is not known in 10 children.

168 (70.6%) children recorded no bleed during year 2021.



Location of bleeds in 2021

108 (23.0%) adults experienced bleeding requiring treatment at least once in year; 354 bleeds were recorded in total, 12 bleeds required hospitalization. 105 of these 108 adults have recorded location of their bleeds. Localization is not known in 3 adults. 361 (77.0%) adults have recorded no bleed during year 2021.

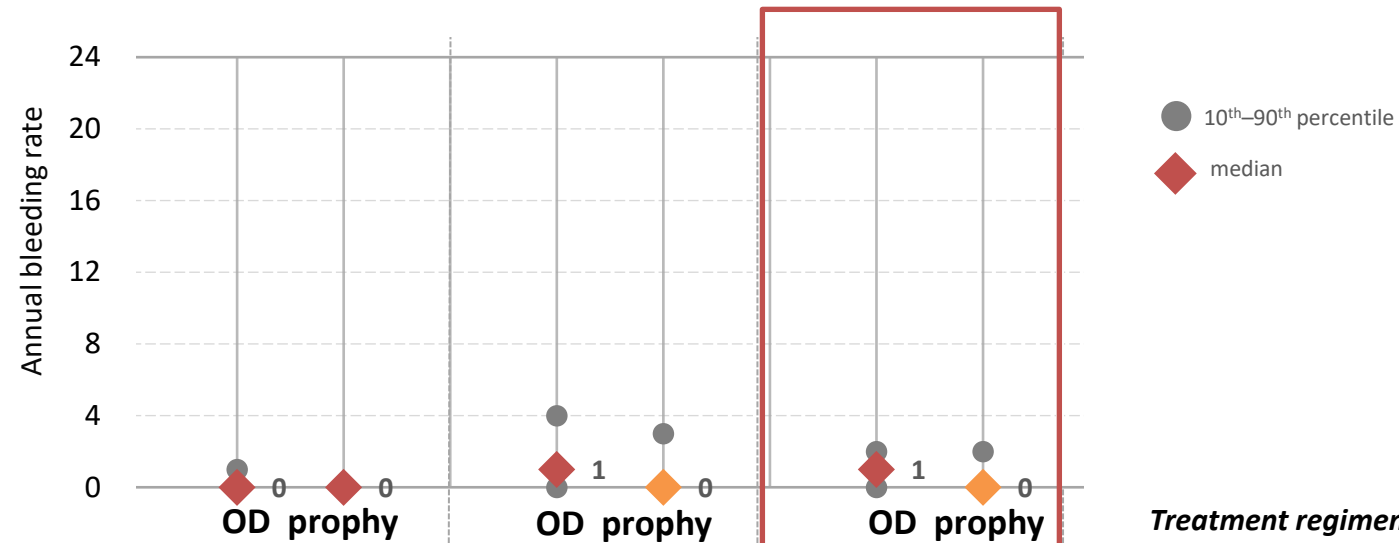


¹Frequency of bleeding is missing in 1 adult.

ABR according to treatment regimen Haemophilia A without inhibitor

Annual bleeding rate according to treatment regimen

Children
Haem A
N=228*



Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	116	0	18	8	13	73
Mean	0.2		1.6	1.1	0.8	1.0
Median (min – max)	0 (0 – 4)		1 (0 – 13)	0 (0 – 3)	1 (0 – 2)	0 (0 – 7)
Total no of recorded bleeds	21		29	9	11	73
children on permanent prophylaxis	0 (0%)		8 (30.8%)		73 (84.9%)	
% of factor (FVIII) consumed by children on permanent prophylaxis	-		93.4%		99.8%	

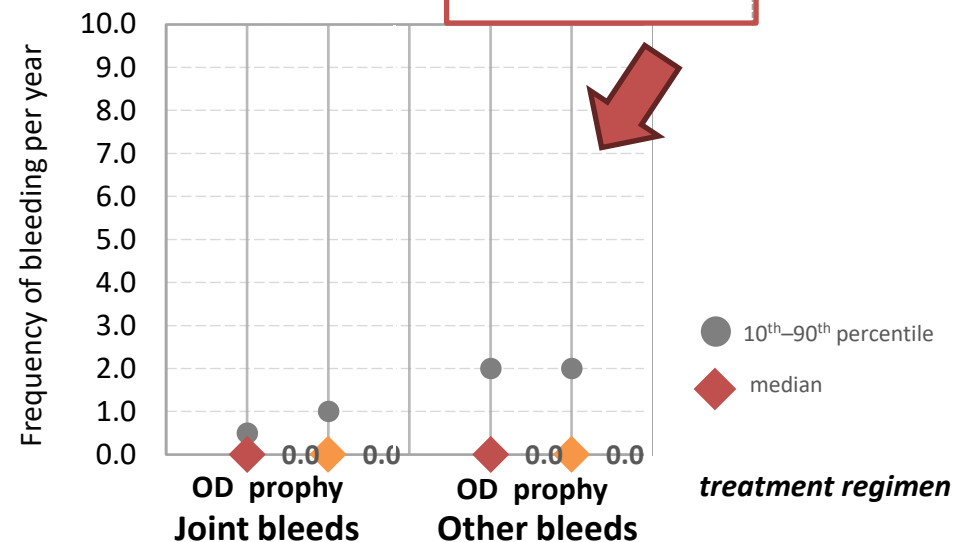
Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

* without inhibitor

Joint and other bleeds according to treatment regimen

Frequency of bleeding	Mild*		Moderate*		Severe*	
	OD	prophy	OD	prophy	OD	prophy
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	115	0	16	8	10	70
JOINT BLEEDS						
Mean	0.1		0.4	0.6	0.1	0.3
Median (range)	0 (0-1)		0 (0-3)	0 (0-3)	0 (0-1)	0 (0-3)
Total no of recorded bleeds	6		7	5	1	22
OTHER BLEEDS						
Mean	0.1		1.3	0.5	0.6	0.6
Median (range)	0 (0-3)		0 (0-13)	0 (0-2)	0 (0-2)	0 (0-6)
Total no of recorded bleeds	14		20	4	6	45

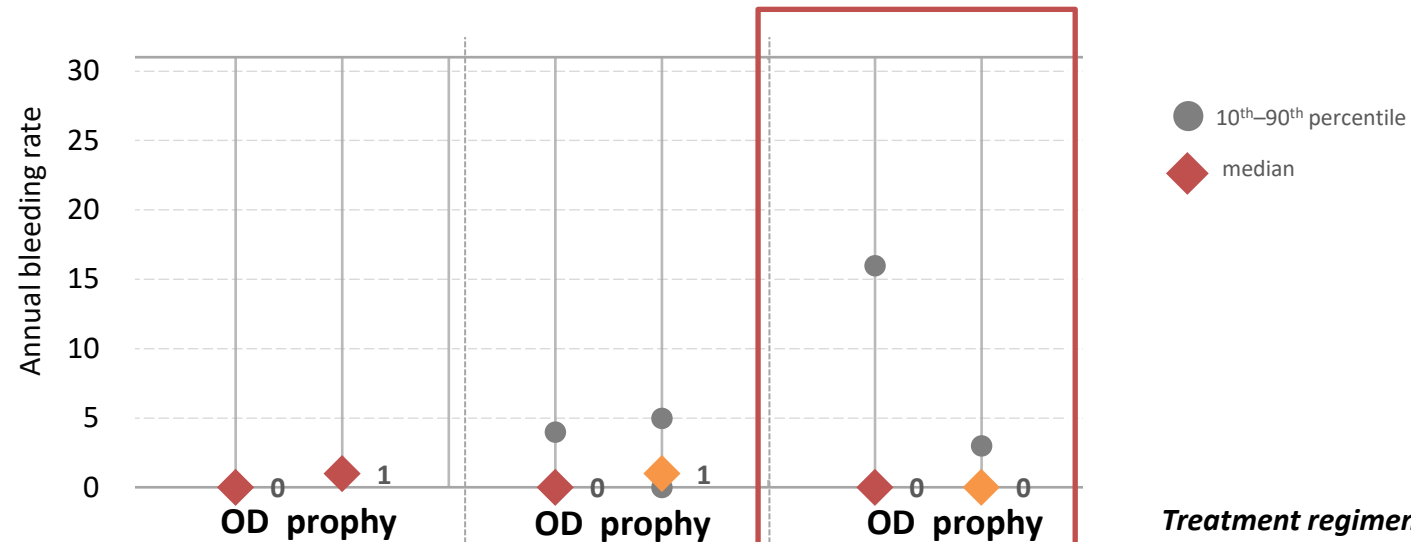
* without inhibitor; missing location of their bleeds in 10 children (one of them is inhibitor)



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

Annual bleeding rate according to treatment regimen

Adults
Haem A
N=460*



Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	260	1	34	3	41	121
Mean	0.1	1.0	1.1	2.0	4.4	0.9
Median (min – max)	0 (0 – 7)	1 (1 – 1)	0 (0 – 7)	1 (0 – 5)	0 (0 – 24)	0 (0 – 7)
Total no of recorded bleeds	34	1	38	6	182	106
adults on permanent prophylaxis	1 (0.4%)		3 (8.1%)		121 (74.2%)	
% of factor (FVIII) consumed by adults on permanent prophylaxis	10.3%		55.3%		95.7%	

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

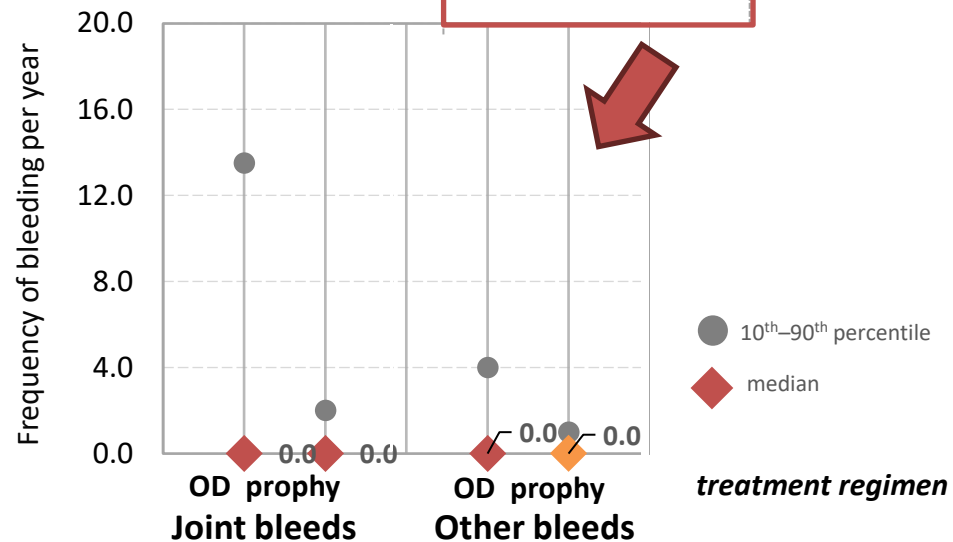
* without inhibitor; missing severity in 2 adults; missing frequency of bleeding in one adult

Joint and other bleeds according to treatment regimen

Adults
Haem A
N=457*

Frequency of bleeding	Mild*		Moderate*		Severe*	
	OD	prophy	OD	prophy	OD	prophy
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	260	1	34	3	40	119
JOINT BLEEDS						
Mean	0.0	1	0.8	0.7	3.1	0.7
Median (range)	0 (0-2)	1 (1-1)	0 (0-5)	0 (0-2)	0 (0-22)	0 (0-7)
Total no of recorded bleeds	8	1	28	2	124	81
OTHER BLEEDS						
Mean	0.1	0	0.3	1.3	1.1	0.2
Median (range)	0 (0-7)	0 (0-0)	0 (0-3)	1 (0-3)	0 (0-10)	0 (0-3)
Total no of recorded bleeds	25	0	10	4	45	22

* without inhibitor; missing severity in 2 adults; missing frequency of bleeding in one adult; missing location of bleeds in 3 adults



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

ABR according to treatment regimen and age

Adults
Haem A
N=460*

* without inhibitor; missing severity in 2 adults ; missing frequency of bleeding in one adult

Frequency of bleeding	Mild*		Moderate*		Severe*		
	OD	Prophy	OD	Prophy	OD	Prophy	
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	Adults (haem A) born <u>before 1990</u> N=333
N valid	194	0	21	2	34	82	
Mean	0.1		1.0	3.0	5.3	0.9	
Median (min – max)	0 (0 – 7)		0 (0 – 7)	3 (1 – 5)	0 (0 – 24)	0 (0 – 7)	
Total no of recorded bleeds	24		21	6	180	73	
adults on permanent prophylaxis	0 (0%)		2 (8.7%)		82 (70.1%)		
% of factor (FVIII) consumed by adults on permanent prophylaxis	-		47.7%		94.7%		
Frequency of bleeding	Mild*		Moderate*		Severe*		
	OD	Prophy	OD	Prophy	OD	Prophy	
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	Adults (haem A) born in <u>1990 or later</u> N=127
N valid	66	1	13	1	7	39	
Mean	0.2	1.0	1.3	0.0	0.3	0.8	
Median (min – max)	0 (0 – 2)	1 (1 – 1)	0 (0 – 5)	0 (0 – 0)	0 (0 – 1)	0 (0 – 7)	
Total no of recorded bleeds	10	1	17	0	2	33	
adults on permanent prophylaxis	1 (1.5%)		1 (7.1%)		39 (84.8%)		
% of factor (FVIII) consumed by adults on permanent prophylaxis	28.2%		68.8%		97.8%		

Adults
Haem A
N=457*

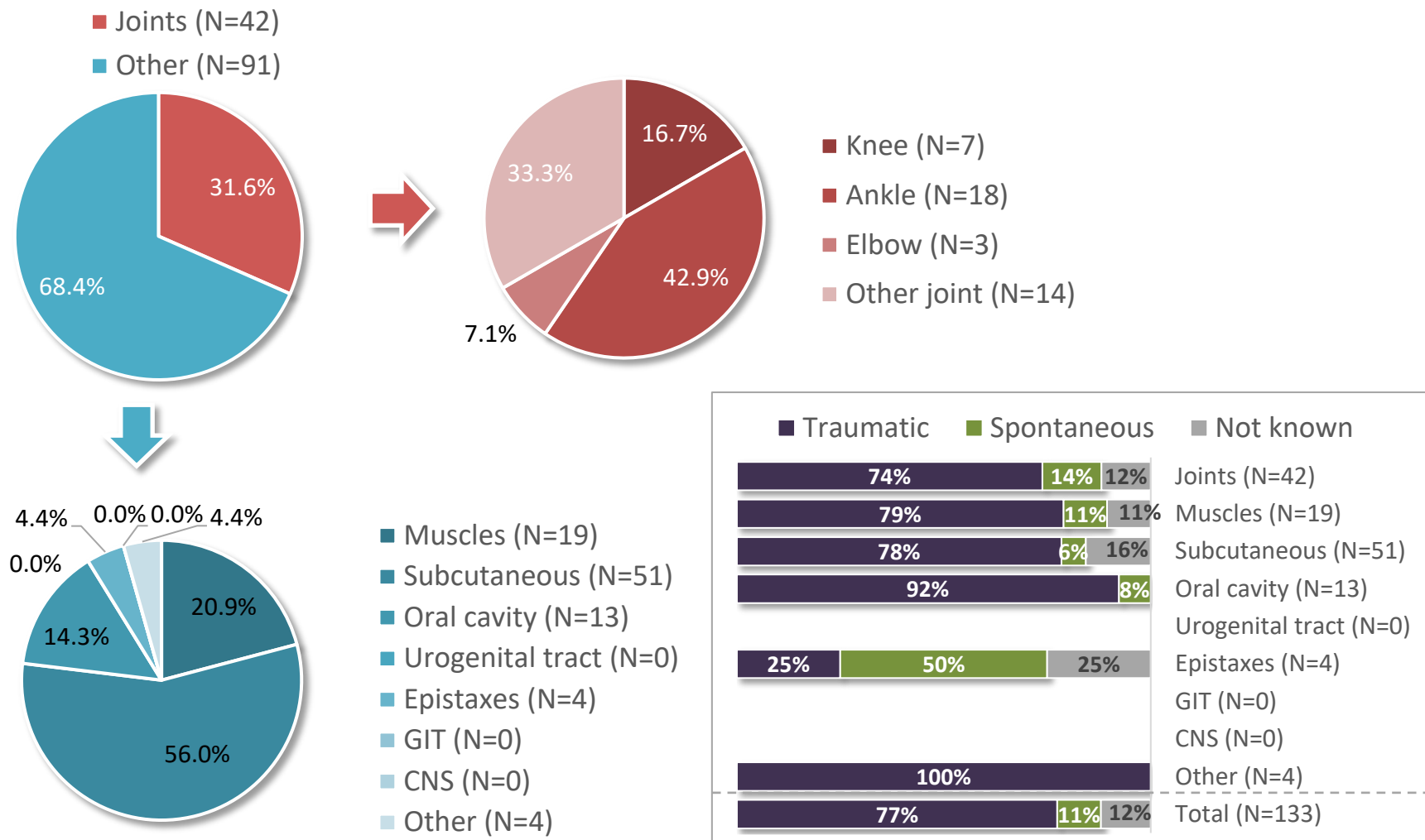
Joint and other bleeds according to treatment regimen and age

* without inhibitor; missing severity in 2 adults; missing frequency of bleeding in one adult; missing location of bleeds in 3 adults

Frequency of bleeding	Mild*		Moderate*		Severe*		
Treatment regimen	OD	prophy	OD	prophy	OD	prophy	
N valid	194	0	21	2	33	81	
JOINT BLEEDS							
Mean	0.0		0.8	1.0	3.7	0.7	Adults (haem A) born <u>before 1990</u> N=331
Median (range)	0 (0-2)		0 (0-5)	1 (0-2)	0 (0-22)	0 (0-7)	
Total no of recorded bleeds	6		17	2	123	57	
OTHER BLEEDS							
Mean	0.1		0.2	2.0	1.3	0.2	Adults (haem A) born in <u>1990 or later</u> N=126
Median (range)	0 (0-7)		0 (0-2)	2 (1-3)	0 (0-10)	0 (0-2)	
Total no of recorded bleeds	17		4	4	44	14	

Frequency of bleeding	Mild*		Moderate*		Severe*		
Treatment regimen	OD	prophy	OD	prophy	OD	prophy	
N valid	66	1	13	1	7	38	
JOINT BLEEDS							
Mean	0.0	1	0.8	0.0	0.1	0.6	Adults (haem A) born in <u>1990 or later</u> N=126
Median (range)	0 (0-1)	1 (1-1)	0 (0-5)	0 (0-0)	0 (0-1)	0 (0-7)	
Total no of recorded bleeds	2	1	11	0	1	24	
OTHER BLEEDS							
Mean	0.1	0	0.5	0.0	0.1	0.2	Adults (haem A) born in <u>1990 or later</u> N=126
Median (range)	0 (0-2)	0 (0-0)	0 (0-3)	0 (0-0)	0 (0-1)	0 (0-3)	
Total no of recorded bleeds	8	0	6	0	1	8	

Location and etiology of bleeds



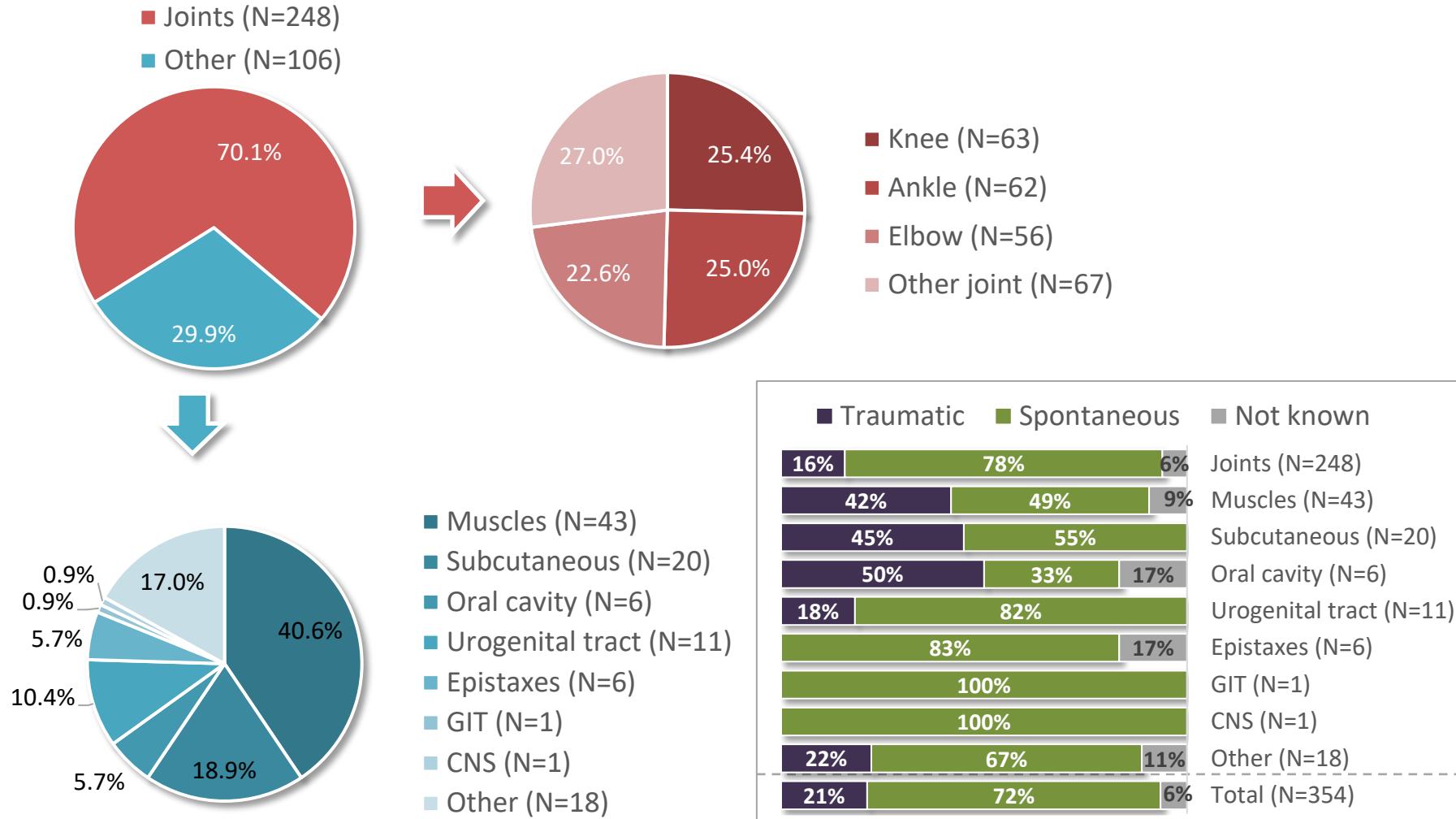
Detailed treatment of bleeds

* number of bleeds

	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	42	19	51	13	0	4	0	0	4	133
FVIII consumption per bleed (IU), valid N	42	19	51	13	0	4	0	0	4	133
geometric mean	2134.1	2242.7	1053.2	1032.7	1.0	840.9	1.0	1.0	2201.7	1486.4
median	1500.0	2000.0	1000.0	1000.0	0.0	1000.0	0.0	0.0	2175.0	1500.0
min – max	500–20000	250–36000	125–10000	250–4500	0–0	500–1000	0–0	0–0	1000–5000	125–36000
sum	144500	106000	74875	17550	0	3500	0	0	10350	356775
No. of doses per bleed										
geometric mean	1.6	1.8	1.3	1.3	1.0	1.0	1.0	1.0	1.4	1.5
median	1	1	1	1	0	1	0	0	1	1
min – max	1–13	1–18	1–5	1–6	0–0	1–1	0–0	0–0	1–4	1–18
Duration of therapy per bleed, days										
geometric mean	1.8	1.9	1.3	1.6	1.0	1.0	1.0	1.0	2.4	1.6
median	1	1	1	1	0	1	0	0	3	1
min – max	1–18	1–31	1–17	1–5	0–0	1–1	0–0	0–0	1–9	1–31
N (%) with hospitalization	5 (11.9%)	0 (0%)	2 (3.9%)	3 (23.1%)	0 (0%)	1 (25%)	0 (0%)	0 (0%)	1 (25%)	12 (9%)
N (%) with rebleeding	2 (4.8%)	0 (0%)	1 (2%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	3 (2.3%)

* number of bleeds

Location and etiology of bleeds



Adults
Haem A
N=354*

Detailed treatment of bleeds

* number of bleeds

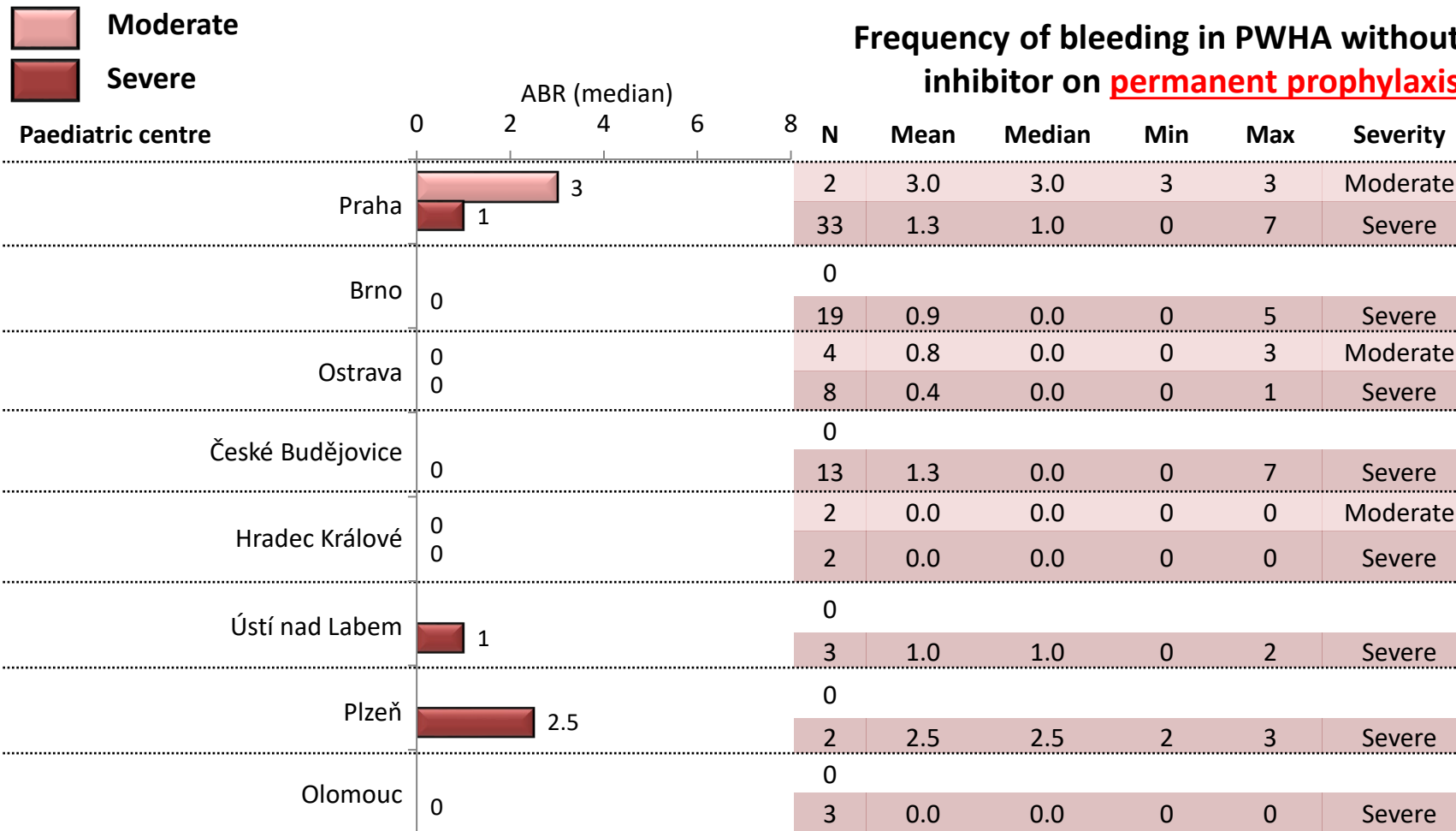
	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	248	43	20	6	11	6	1	1	18	354
FVIII consumption per bleed (IU), valid N	247	43	20	6	11	6	1	1	18	353
geometric mean	2238.2	2554.7	3650.7	2542.3	5410.7	3989.5	15000.0	68500.0	1737.3	2438.0
median	2000.0	2000.0	3000.0	2500.0	10000.0	3000.0	15000.0	68500.0	2000.0	2000.0
min – max	500–151000	500–33000	500–78000	1000–10000	1000–24000	2000–14000	15000–15000	68500–68500	500–9500	500–151000
sum	1298000	207750	163000	20500	112000	33000	15000	68500	52500	1970250
No. of doses per bleed										
geometric mean	1.8	2.0	2.1	1.4	4.7	1.7	5.0	53.0	1.4	1.9
median	1	2	1	1	6	1	5	53	1	1
min – max	1–70	1–16	1–42	1–4	1–24	1–7	5–5	53–53	1–6	1–70
Duration of therapy per bleed, days										
geometric mean	1.6	1.8	2.1	1.4	3.7	1.7	9.0	23.0	1.5	1.7
median	1	1	1	1	5	1	9	23	1	1
min – max	1–30	1–15	1–46	1–4	1–14	1–7	9–9	23–23	1–13	1–46
N (%) with hospitalization	7 (2.8%)	0 (0%)	0 (0%)	0 (0%)	2 (18.2%)	0 (0%)	0 (0%)	1 (100%)	2 (11.1%)	12 (3.4%)
N (%) with rebleeding	18 (7.3%)	0 (0%)	0 (0%)	1 (16.7%)	2 (18.2%)	0 (0%)	0 (0%)	0 (0%)	1 (5.6%)	22 (6.2%)

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

ABR according to centres Haemophilia A (PWHA)

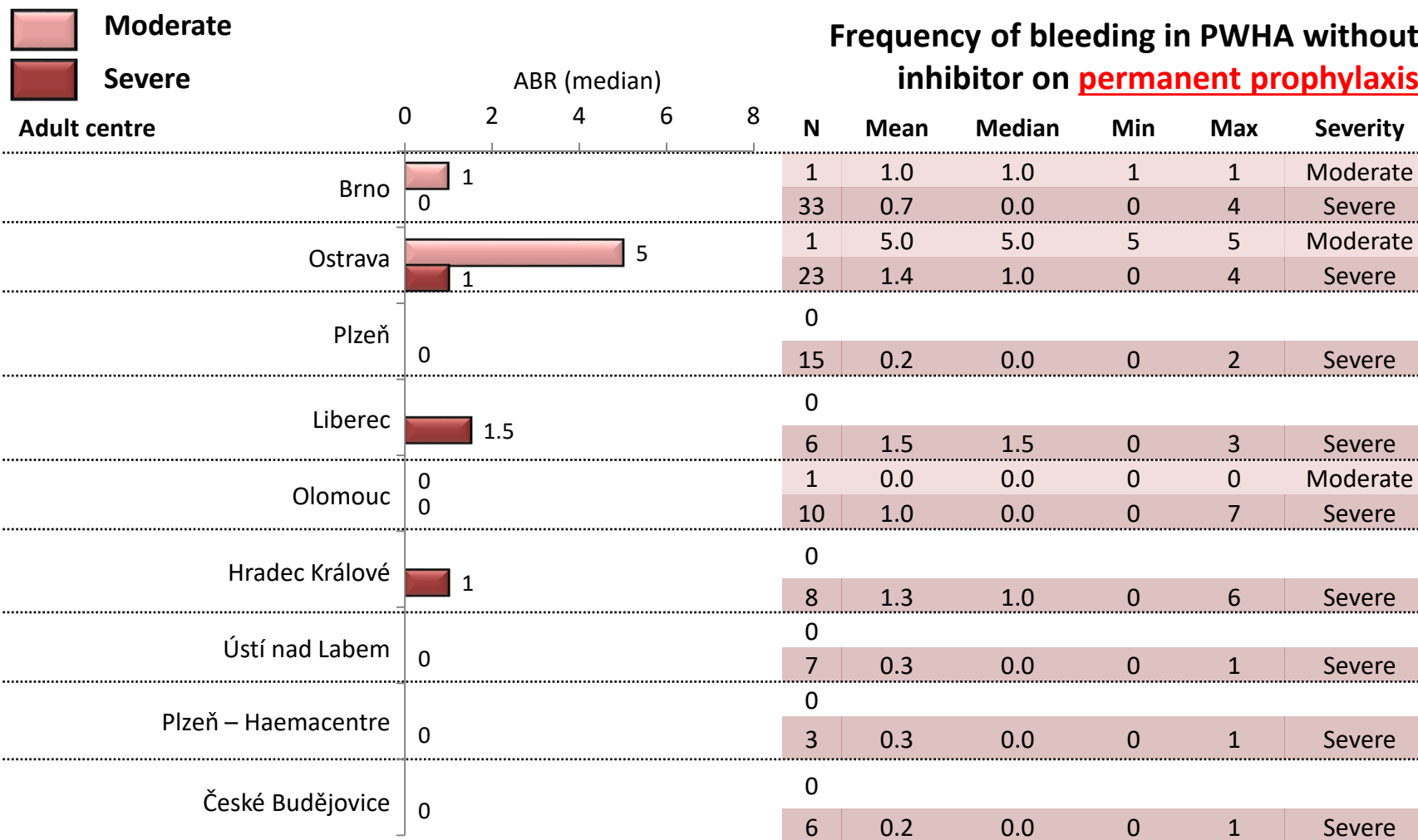
Annual bleeding rate on permanent prophylaxis

HaemA on prophylaxis
Paed. centres
N=91



Annual bleeding rate on permanent prophylaxis

HaemA on prophylaxis
Adult centres
N=114



Annual bleeding rate regardless prophylaxis

HaemA
Paed. centres
N=122



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

Paediatric centre	ABR (median)		N	Mean	Median	Min	Max	% on permanent prophylaxis
	0	2						
Praha	2	1	8	1.8	2.0	0	3	25.0%
	0	1	39	1.3	1.0	0	7	84.6%
Brno	0	1	5	0.4	0.0	0	1	0.0%
	0	0	23	1.0	1.0	0	5	82.6%
Ostrava	0	0	4	0.8	0.0	0	3	100.0%
	0	0	8	0.4	0.0	0	1	100.0%
České Budějovice	2.5	0	2	2.5	2.5	1	4	0.0%
	0	0	13	1.3	0.0	0	7	100.0%
Hradec Králové	0	0	2	0.0	0.0	0	0	100.0%
	0	0	2	0.0	0.0	0	0	100.0%
Ústí nad Labem	0	1	1	0.0	0.0	0	0	0.0%
	0	1	3	1.0	1.0	0	2	100.0%
Plzeň	0	1	1	0.0	0.0	0	0	0.0%
	0	1	4	1.3	1.0	0	3	50.0%
Olomouc	1	0	3	4.7	1.0	0	13	0.0%
	0	0	4	0.0	0.0	0	0	75.0%

Annual bleeding rate regardless prophylaxis

HaemA
Adult centres
N=189*

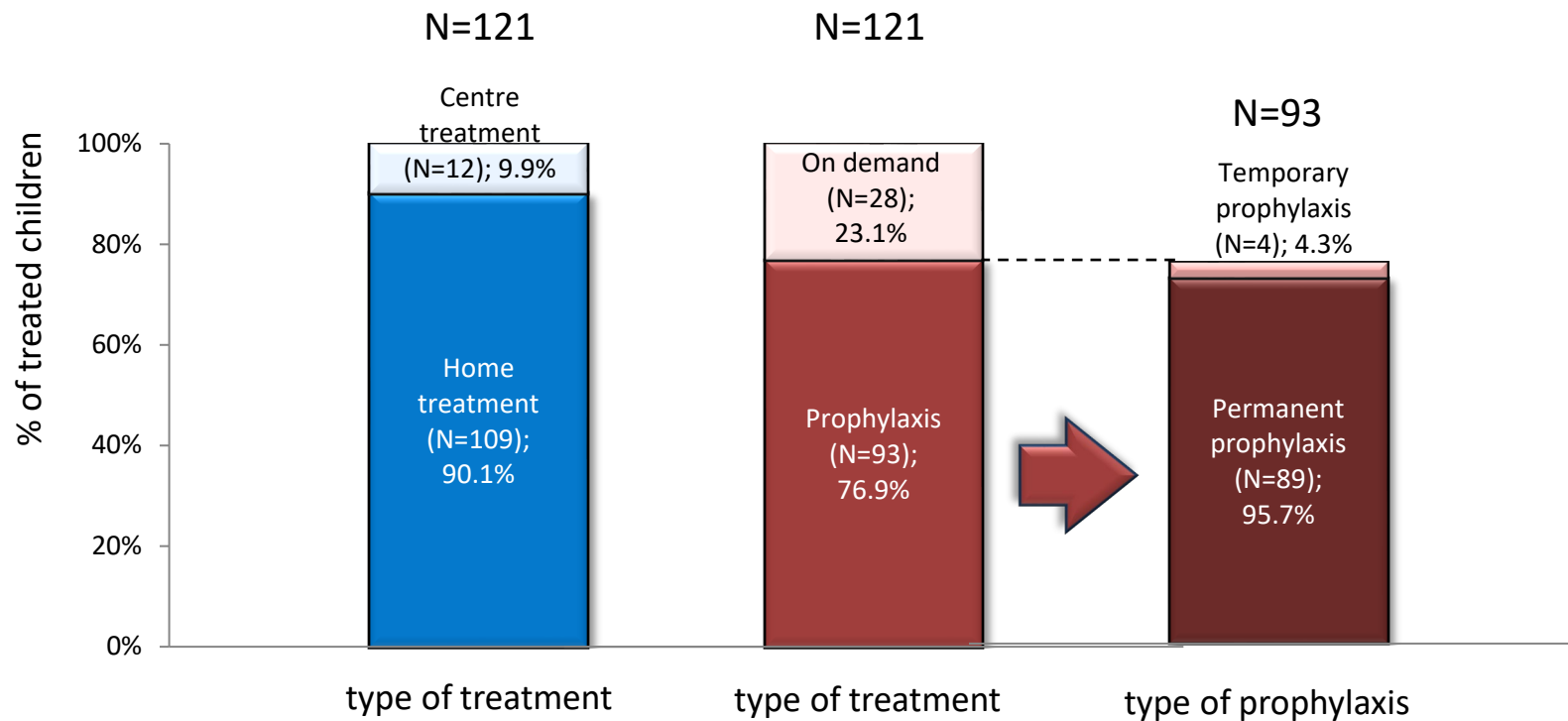
* missing ABR in 1 adult



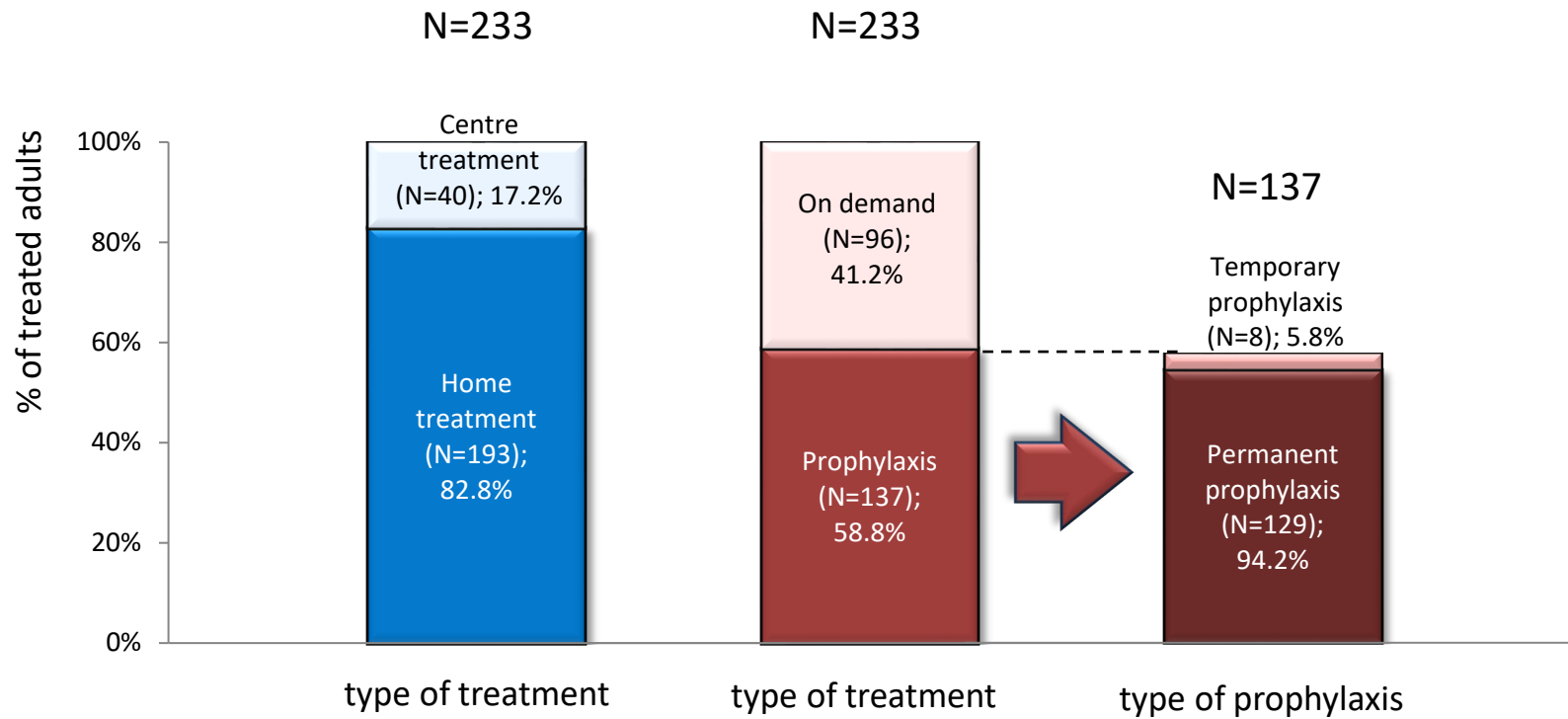
Frequency of bleeding in PWHA without inhibitor regardless of prophylaxis

Adult centre	ABR (median)					N	Mean	Median	Min	Max	% on permanent prophylaxis
	0	2	4	6	8						
Brno	0.0					13	0.3	0.0	0	1	7.7%
	0.0					41	0.7	0.0	0	4	80.5%
Ostrava			5.0			7	3.3	5.0	0	7	14.3%
		1.0				25	1.3	1.0	0	4	92.0%
Plzeň	0.0					3	0.0	0.0	0	0	0.0%
	0.0					19	0.2	0.0	0	2	75.0%
Liberec			3.0			1	3.0	3.0	3	3	0.0%
			2.0			10	5.3	2.0	0	24	60.0%
Olomouc			2.0			2	2.0	2.0	0	4	50.0%
	0.0					20	6.3	0.0	0	22	50.0%
Hradec Králové	0.0					5	0.6	0.0	0	2	0.0%
	0.0					16	1.4	0.0	0	13	50.0%
Ústí nad Labem	0.0					3	0.0	0.0	0	0	0.0%
	0.0					7	0.3	0.0	0	1	100.0%
Plzeň – Haemacentre			3.0			1	3.0	3.0	3	3	0.0%
	0.0					3	0.3	0.0	0	1	100.0%
České Budějovice			2.0			2	2.0	2.0	0	4	0.0%
	0.0					11	0.5	0.0	0	3	54.5%

Type of treatment (subgroup of treated patients)



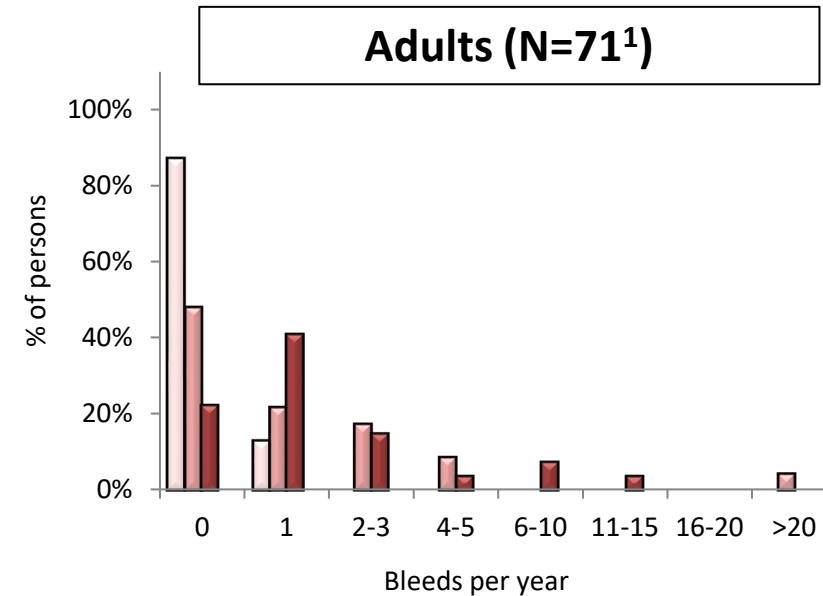
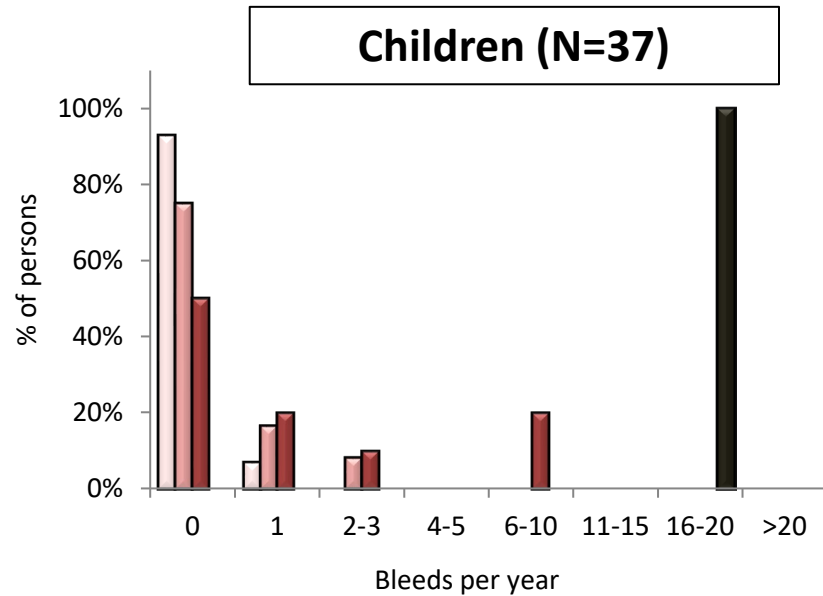
Type of treatment (subgroup of treated patients)



Treatment outcomes and bleeding frequency

Haemophilia B

Frequency of bleeding requiring treatment in 2019



Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
14	12	10	1	N valid	23	23	25	0
0.1	0.4	1.6	17.0	Mean	0.1	5.8	2.2	0.0
0 (0 – 1)	0 (0 – 3)	0.5 (0 – 6)	17 (17 – 17)	Median (min – max)	0 (0 – 1)	1 (0 – 113)	1 (0 – 12)	(–)
13 (92.9%)	9 (75%)	5 (50%)	0 (0%)	N (%) with no bleed	20 (87%)	11 (47.8%)	6 (22.2%)	0 (0%)

* without inhibitor

¹Frequency of bleeding is missing in 2 adults.

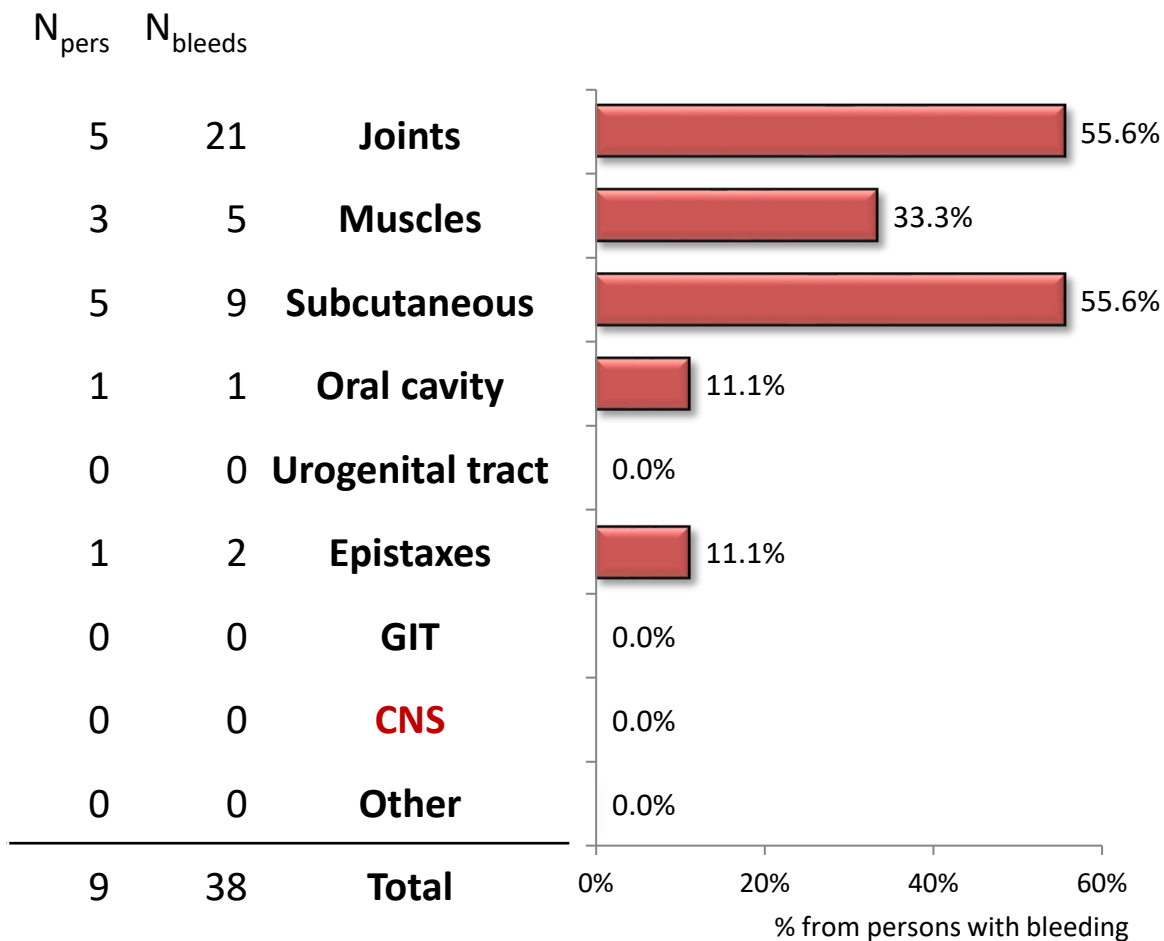
Location of bleeds in 2021

Children
Haem B
N=37

10 (27.0%) children experienced bleeding requiring treatment at least once in year; 38 bleeds were recorded in total, 3 bleeds required hospitalization.

9 of these 10 children have recorded location of their bleeds. Localization is not known in 1 child.

27 (73.0%) children recorded no bleed during year 2021.



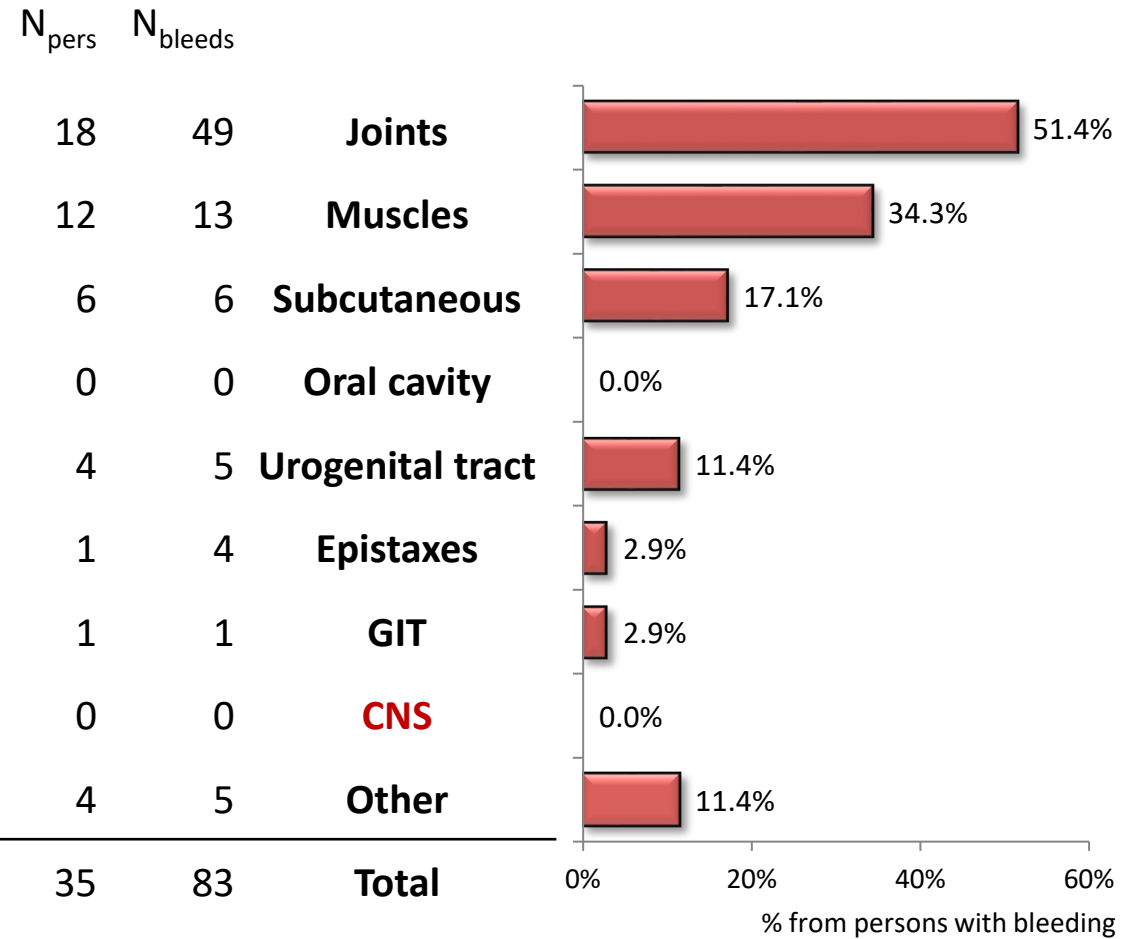
Location of bleeds in 2021

Adults
Haem B
N=71¹

35 (49.3%) adults experienced bleeding requiring treatment at least once in year; 83 bleeds were recorded in total, 9 bleeds required hospitalization.

All of these 35 adults have recorded location of their bleeds.

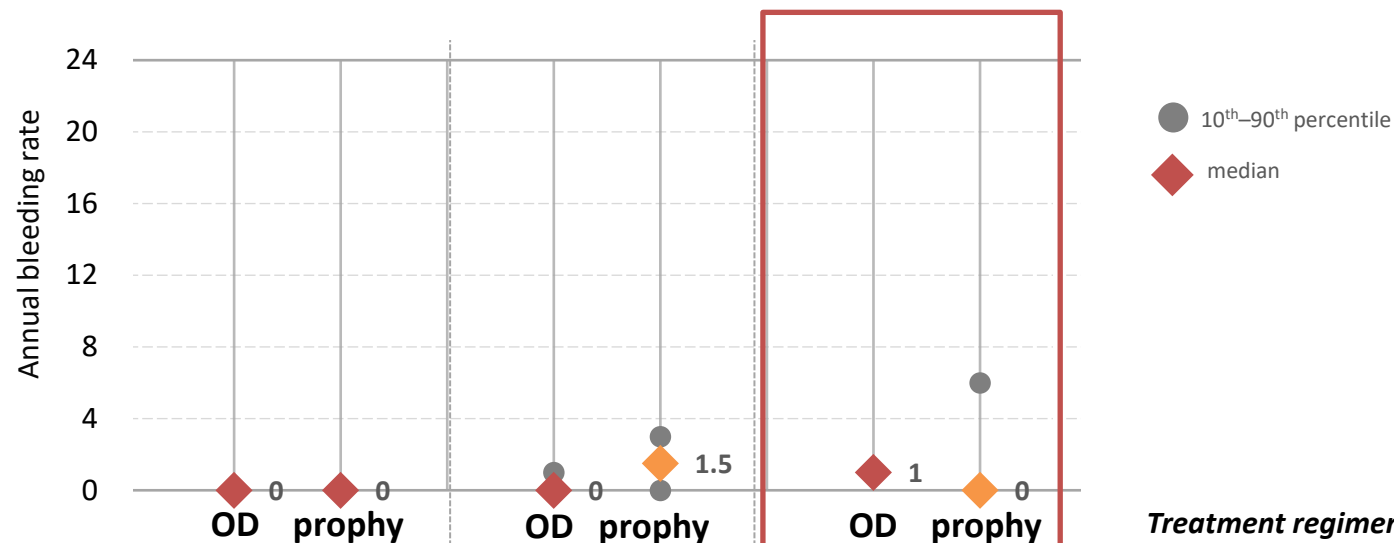
36 (50.7%) adults have recorded no bleed during year 2021.



¹Frequency of bleeding is missing in 2 adults.

ABR according to treatment regimen Haemophilia B without inhibitor

Annual bleeding rate according to treatment regimen



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

* without inhibitor

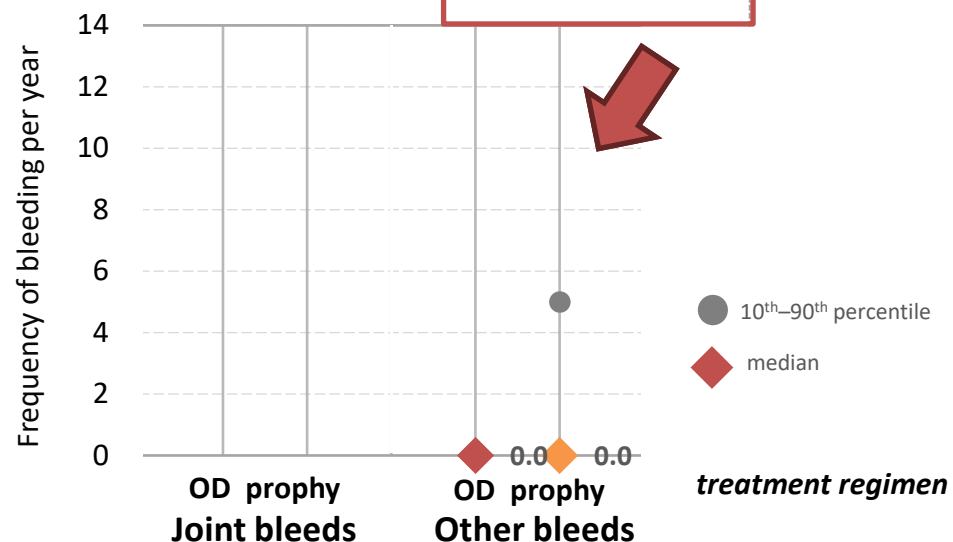
Frequency of bleeding	Mild*		Moderate*		Severe*	
N valid	14	0	10	2	1	9
Mean	0.1		0.2	1.5	1.0	1.7
Median (min – max)	0 (0 – 1)		0 (0 – 1)	1.5 (0 – 3)	1 (1 – 1)	0 (0 – 6)
Total no of recorded bleeds	1		2	3	1	15
children on permanent prophylaxis	0 (0%)		2 (16.7%)		9 (90%)	
% of factor (FIX) consumed by children on permanent prophylaxis	-		99.0%		99.9%	

Joint and other bleeds according to treatment regimen

Children
Haem B
N=35*

* without inhibitor;
missing location of
their bleeds in 1 child

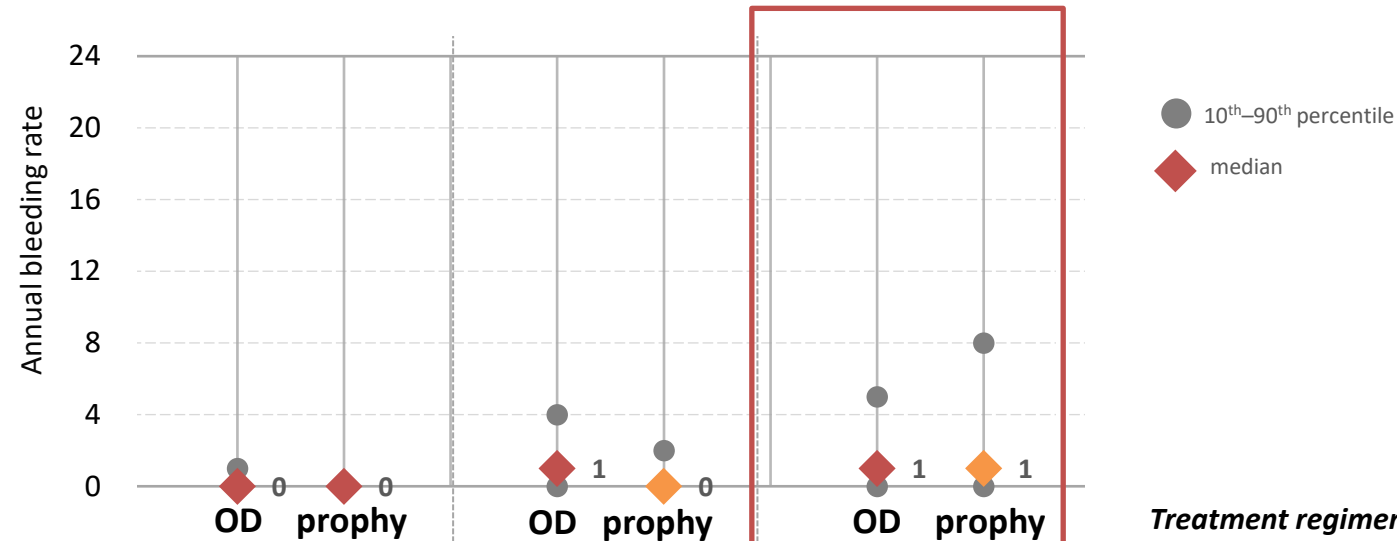
Frequency of bleeding	Mild*		Moderate*		Severe*	
	OD	prophy	OD	prophy	OD	prophy
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	14	0	10	2	0	9
JOINT BLEEDS						
Mean	0.0		0.1	0.5		0.8
Median (range)	0 (0-0)		0 (0-1)	0.5 (0-1)		0 (0-6)
Total no of recorded bleeds	0		1	1		7
OTHER BLEEDS						
Mean	0.1		0.1	1.0		0.9
Median (range)	0 (0-1)		0 (0-1)	1 (0-2)		0 (0-5)
Total no of recorded bleeds	1		1.00	2		8



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

Annual bleeding rate according to treatment regimen

Adults
Haem B
N=71*



Frequency of bleeding	Mild*		Moderate*		Severe*	
N total	23	0	19	4	4	21
Mean	0.1		6.9	0.5	1.8	2.3
Median (min – max)	0 (0 – 1)		1 (0 – 113)	0 (0 – 2)	1 (0 – 5)	1 (0 – 12)
Total no of recorded bleeds	3		132	2	7	49
adults on permanent prophylaxis	0 (0%)		4 (17.4%)		23 (85.2%)	
% of factor (FIX) consumed by adults on permanent prophylaxis	-		64.3%		99.1%	

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

* missing frequency of bleeding in 2 adults

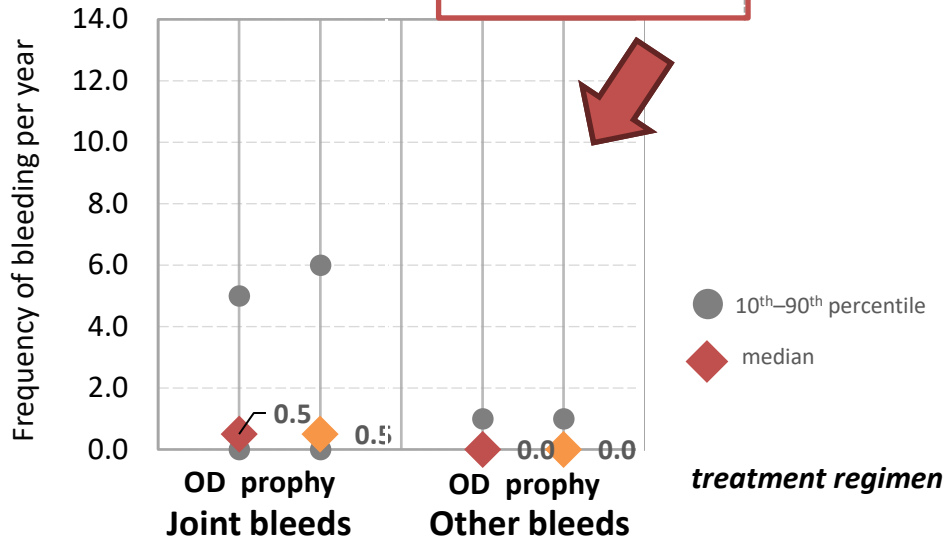
Joint and other bleeds according to treatment regimen

Adults
Haem B
N=72*

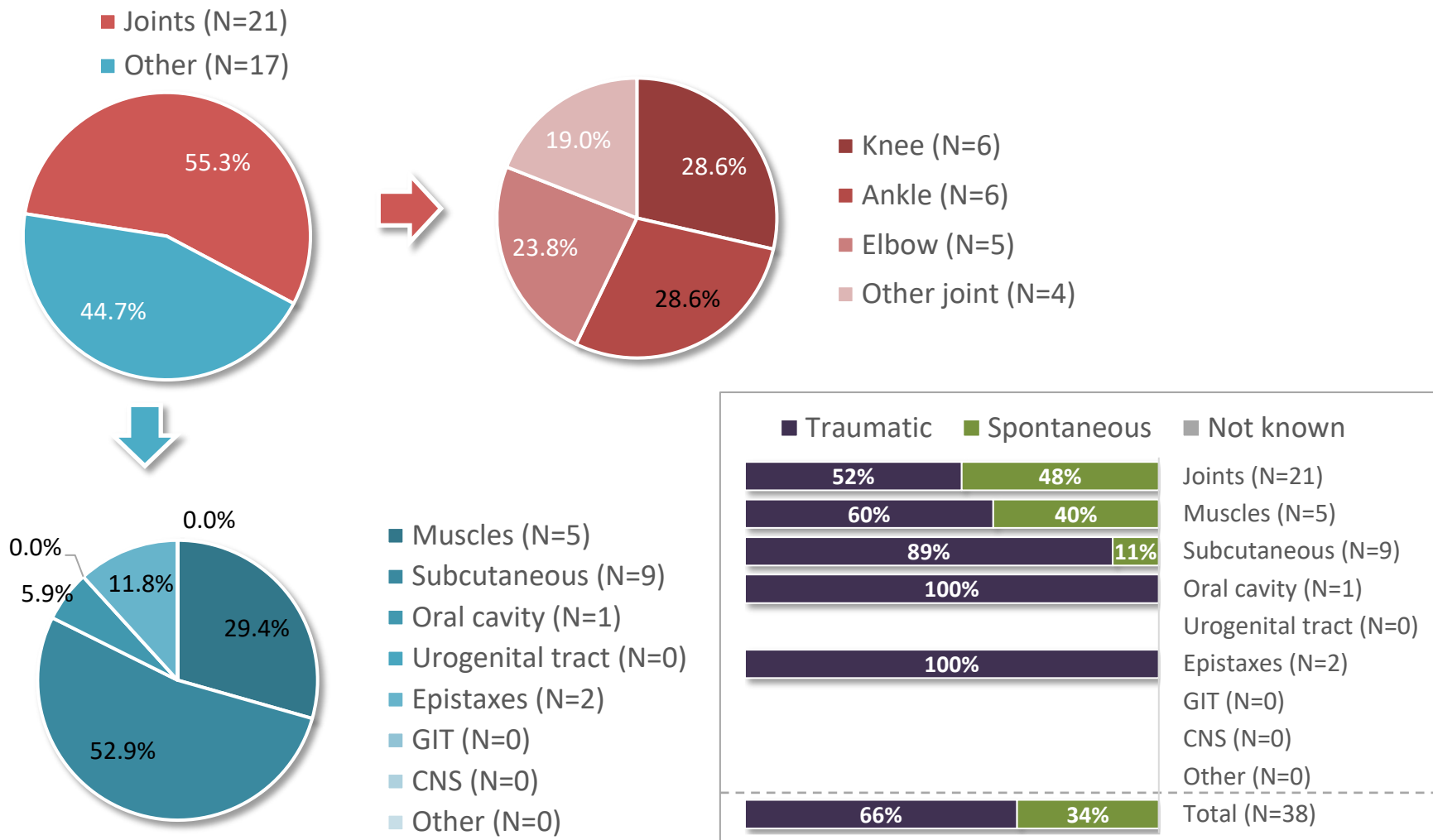
Frequency of bleeding	Mild*		Moderate*		Severe*	
	OD	prophy	OD	prophy	OD	prophy
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	23	0	19	4	4	22
JOINT BLEEDS						
Mean	0.0		0.2	0.5	1.5	1.7
Median (range)	0 (0-1)		0 (0-1)	0 (0-2)	0.5 (0-5)	0.5 (0-10)
Total no of recorded bleeds	1		3	2	6	37
OTHER BLEEDS						
Mean	0.1		0.9	0.0	0.3	0.6
Median (range)	0 (0-1)		1 (0-4)	0 (0-0)	0 (0-1)	0 (0-4)
Total no of recorded bleeds	2		17	0	1	14

* missing frequency of bleeding in 2 adults

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



Location and etiology of bleeds

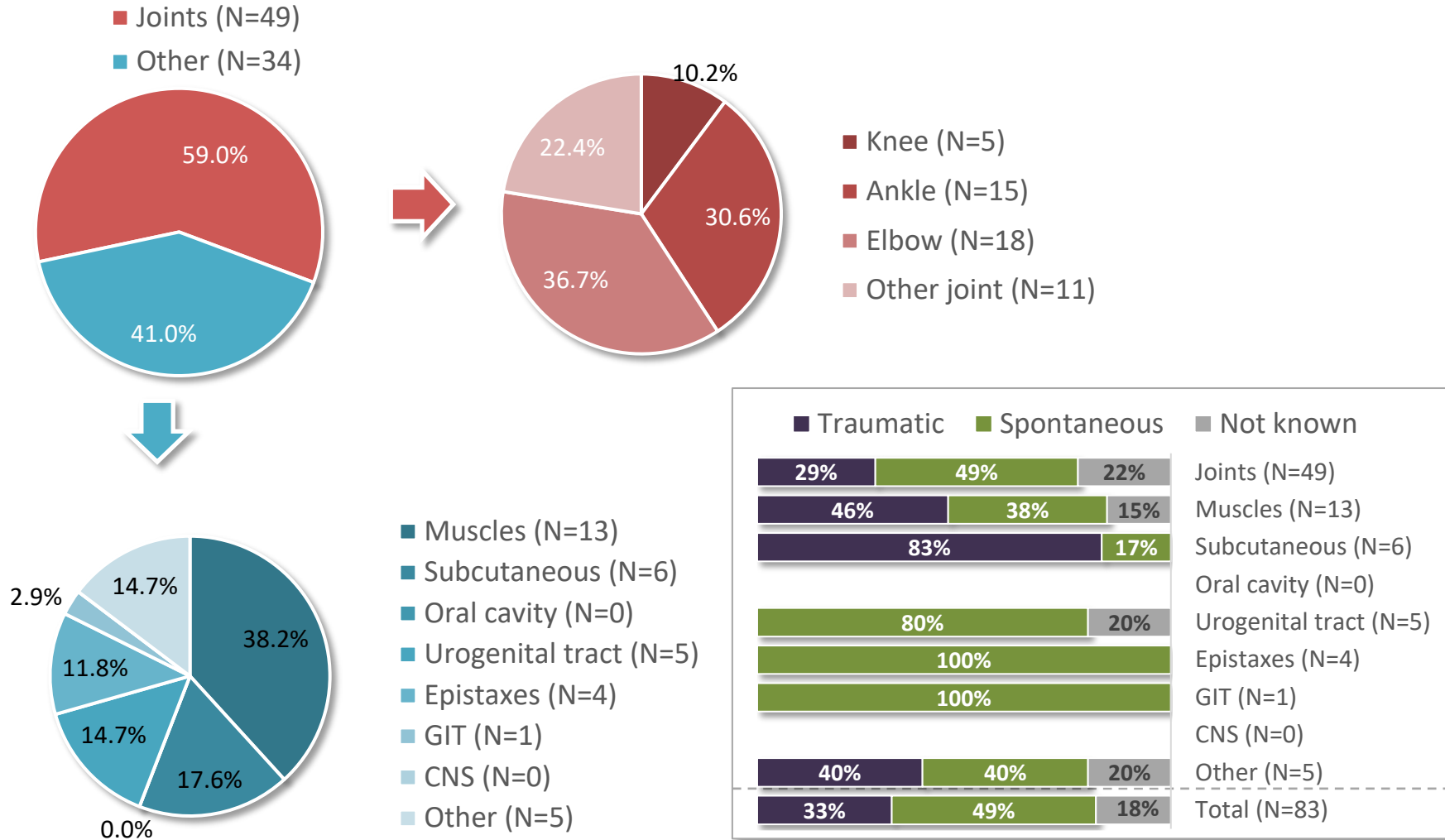


Detailed treatment of bleeds

	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	21	5	9	1	0	2	0	0	0	38
FIX consumption per bleed (IU), valid N	9	3	7	0	0	2	0	0	0	21
geometric mean	2617.1	3693.8	1528.6			500.0				1962.9
median	3000.0	3600.0	2000.0			500.0				3000.0
min – max	500–4000	2000–7000	500–4000			500–500				500–7000
sum	27000	12600	15000			1000				55600
No. of doses per bleed										
geometric mean	1.8	2.4	1.4	6.0		1.0				1.7
median	1	3	1	6		1				1
min – max	1–8	1–4	1–5	6–6		1–1				1–8
Duration of therapy per bleed, days										
geometric mean	1.8	3.1	1.5	8.0		1.0				1.9
median	1	3	1	8		1				1
min – max	1–11	1–9	1–4	8–8		1–1				1–11
N (%) with hospitalization	0 (0%)	1 (20%)	2 (22.2%)	0 (0%)		0 (0%)				3 (7.9%)
N (%) with rebleeding	1 (4.8%)	0 (0%)	0 (0%)	0 (0%)		0 (0%)				1 (2.6%)

* number of bleeds

Location and etiology of bleeds



Adults
Haem B
N=83*

Detailed treatment of bleeds

* number of bleeds

	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	49	13	6	0	5	4	1	0	5	83
FIX consumption per bleed (IU), valid N	49	13	6	0	5	4	1	0	5	83
geometric mean	4861.0	6866.4	8098.0		6252.4	4950.0	56000.0		2845.7	5395.0
median	4000.0	4000.0	10000.0		4800.0	4950.0	56000.0		3000.0	4000.0
min – max	2000–33000	1000–87000	2400–24000		3600–19200	4950–4950	56000–56000		1200–4800	1000–87000
sum	311200	248800	66400		38400	19800	56000		15600	756200
No. of doses per bleed										
geometric mean	1.6	3.0	2.3		2.7	1.0	44.0		2.3	1.9
median	1	2	2		2	1	44		2	1
min – max	1–17	1–22	1–20		1–12	1–1	44–44		1–4	1–44
Duration of therapy per bleed, days										
geometric mean	1.9	2.7	3.0		2.3	1.0	24.0		2.1	2.1
median	1	2	4		2	1	24		2	1
min – max	1–60	1–16	1–11		1–6	1–1	24–24		1–5	1–60
N (%) with hospitalization	2 (4.1%)	3 (23.1%)	2 (33.3%)		1 (20%)	0 (0%)	1 (100%)		0 (0%)	9 (10.8%)
N (%) with rebleeding	1 (2%)	0 (0%)	1 (16.7%)		0 (0%)	0 (0%)	0 (0%)		1 (20%)	3 (3.6%)

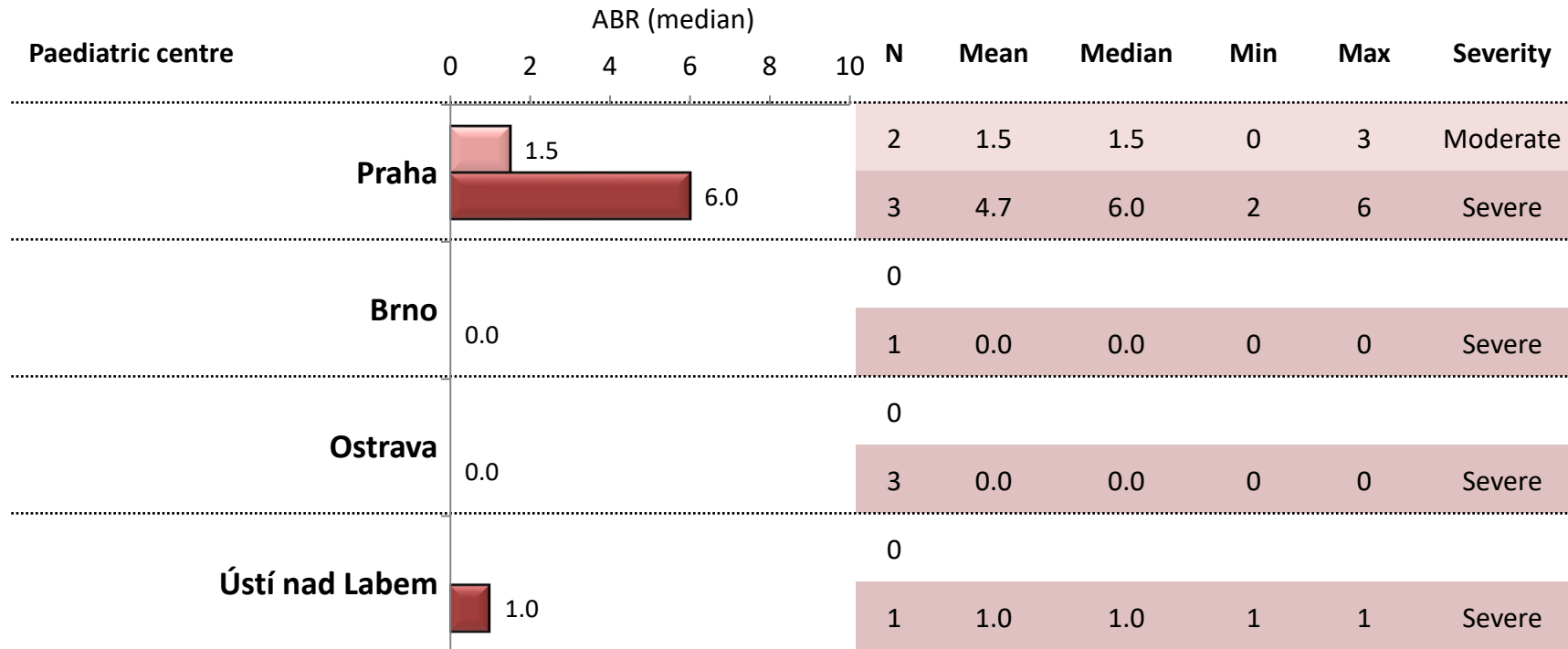
ABR according to centres Haemophilia B (PWHB)

Annual bleeding rate on permanent prophylaxis

HaemB on prophylaxis
Paed. centres
N=10

 Moderate
 Severe

Frequency of bleeding in PWHB without inhibitor on permanent prophylaxis

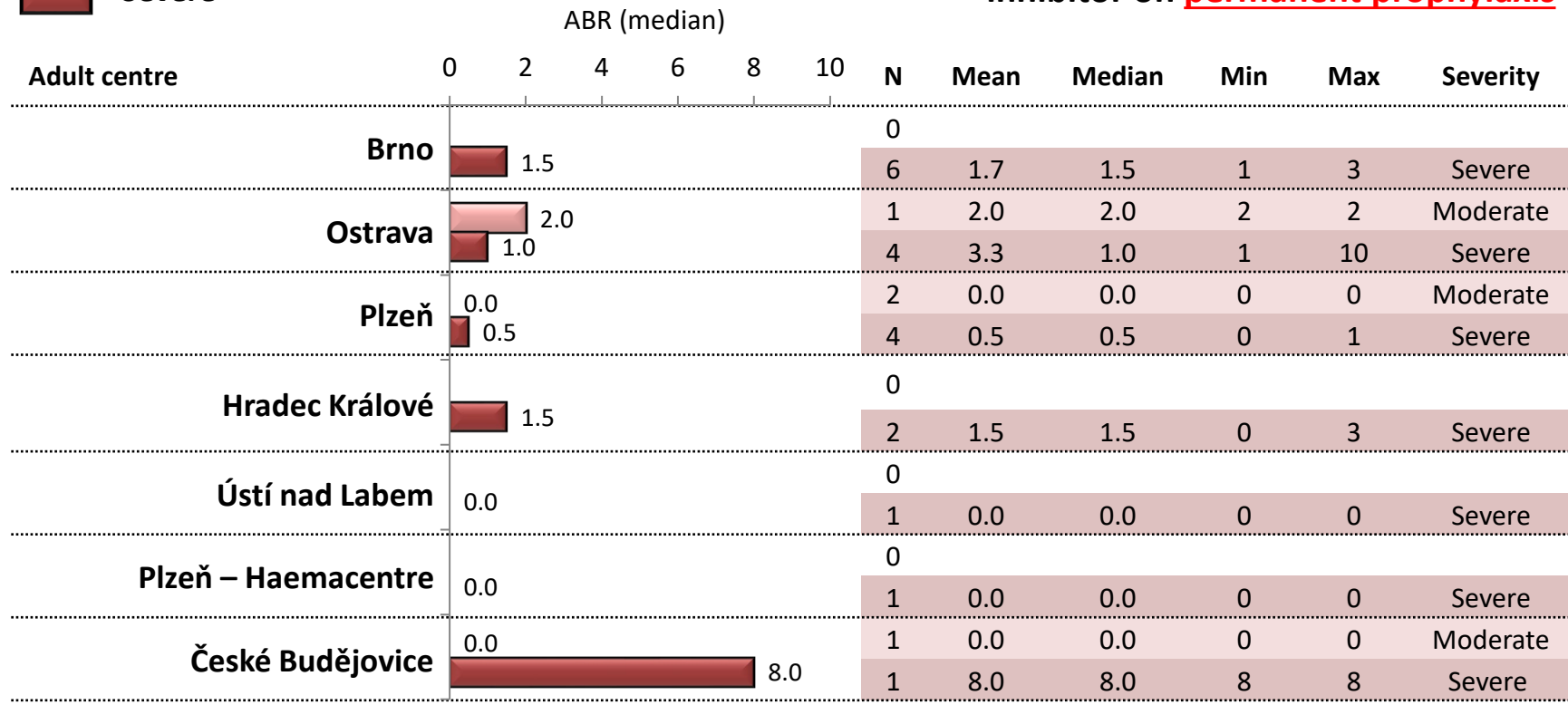


Annual bleeding rate on permanent prophylaxis

HaemB on prophylaxis
Adult centres
N=23

 Moderate
 Severe

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



Annual bleeding rate regardless prophylaxis

HaemB
Paed. centres
N=23

 Moderate
 Severe

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

Paediatric centre	ABR (median)					N	Mean	Median	Min	Max	% on permanent prophylaxis
	0	2	4	6	8						
Praha	0.0					7	0.4	0.0	0	3	28.6%
				6.0		3	4.7	6.0	2	6	100.0%
Brno	0.5					2	0.5	0.5	0	1	0.0%
						2	0.5	0.5	0	1	50.0%
Ostrava	0.0					0					
						3	0.0	0.0	0	0	100.0%
České Budějovice	0.0					1	0.0	0.0	0	0	0.0%
						0					
Hradec Králové	0.0					1	0.0	0.0	0	0	0.0%
						0					
Ústí nad Labem						0					
		1.0				1	1.0	1.0	1	1	100.0%
Plzeň	0.0					1	0.0	0.0	0	0	0.0%
	0.0					1	0.0	0.0	0	0	100.0%
Olomouc						1	1.0	1.0	1	1	0.0%
						0					

Annual bleeding rate regardless prophylaxis

HaemB
Adult centres
N=47*

* missing ABR in 2 adults



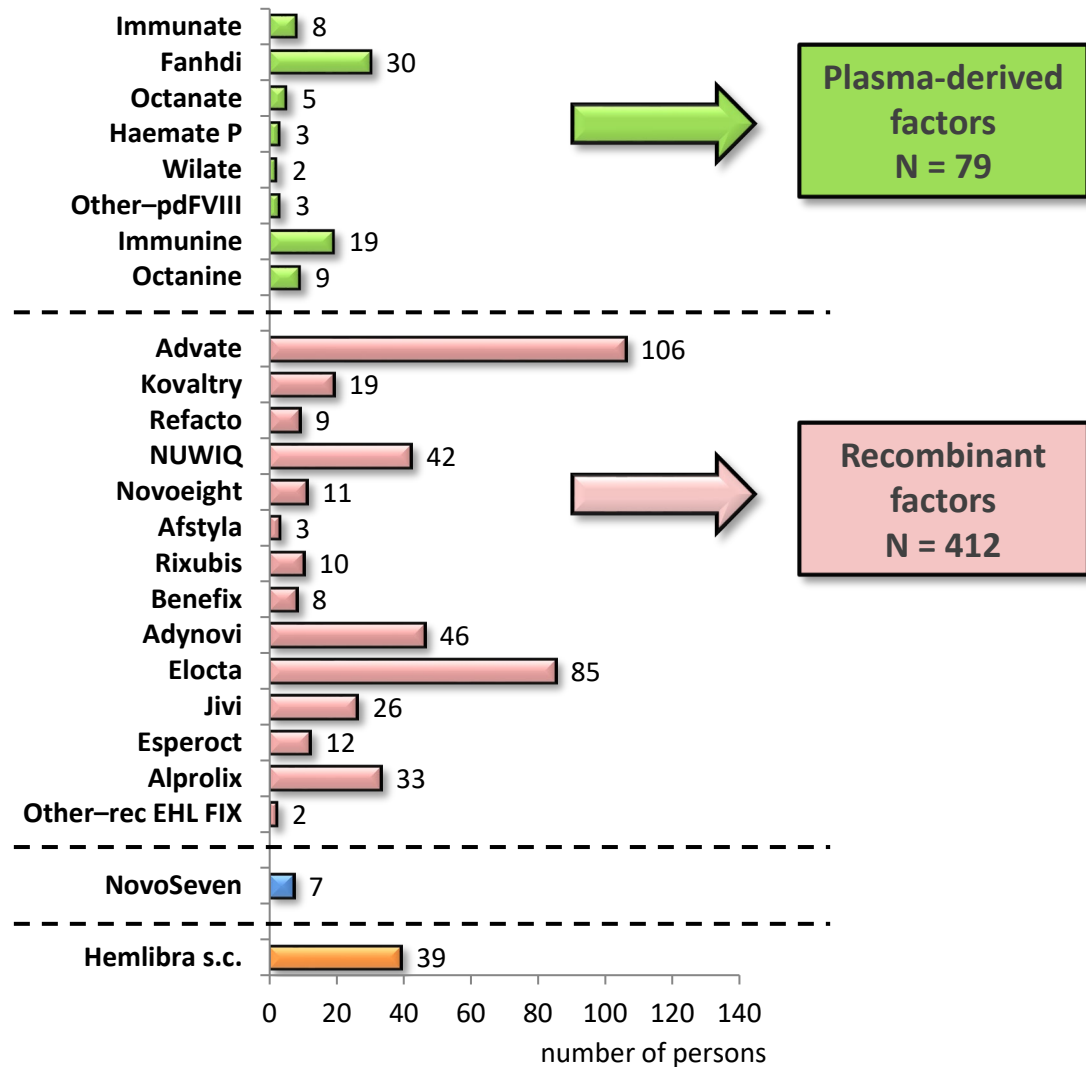
Frequency of bleeding in PWHB without inhibitor **regardless of prophylaxis**

Adult centre	ABR (median)				N*	Mean	Median	Min	Max	% on permanent prophylaxis
	0	5	10	15						
Brno	0.5				4	1.3	0.5	0	4	0.0%
	1.5				6	1.7	1.5	1	3	100.0%
Ostrava	1.0				3	1.0	1.0	0	2	33.3%
	1.0				6	2.5	1.0	1	10	66.7%
Plzeň	0.0				3	0.0	0.0	0	0	66.7%
	0.5				4	0.5	0.5	0	1	100.0%
Liberec	1.0				1	1.0	1.0	1	1	0.0%
	12.0				1	12.0	12.0	12	12	100.0%
Olomouc	2.0				8	15.6	2.0	0	113	0.0%
	1.0				1	1.0	1.0	1	1	100.0%
Hradec Králové	0.0				1	0.0	0.0	0	0	0.0%
	1.5				2	1.5	1.5	0	3	100.0%
Ústí nad Labem					0					
	2.5				2	2.5	2.5	0	5	50.0%
Plzeň – Haemacentre					0					
	0.0				1	0.0	0.0	0	0	100.0%
České Budějovice	0.0				2	0.0	0.0	0	0	50.0%
	4.0				2	4.0	4.0	0	8	50.0%

Treatment data and factor consumption

Haemophilia A and B

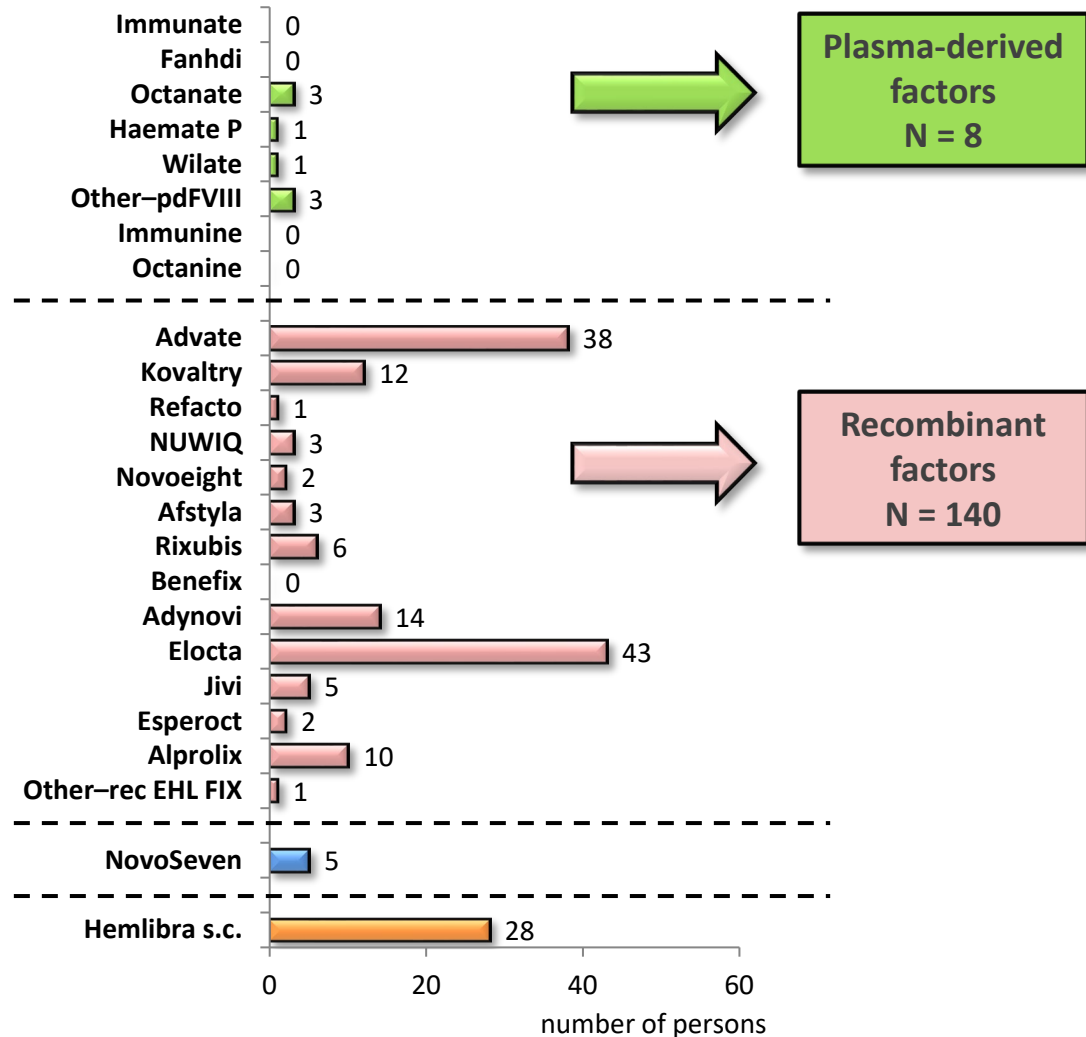
Treatment



422 persons (51.5% of all PWH) were treated in 2021 (**264 persons received standard factor concentrates**, 157 persons received EHL factors, by-pass therapy or emicizumab, in 1 persons data are not available; 94 persons received more than one type/brand of concentrate). 9 persons were treated with both plasma-derived and recombinant factor.

¹missing type of treatment in 1 child

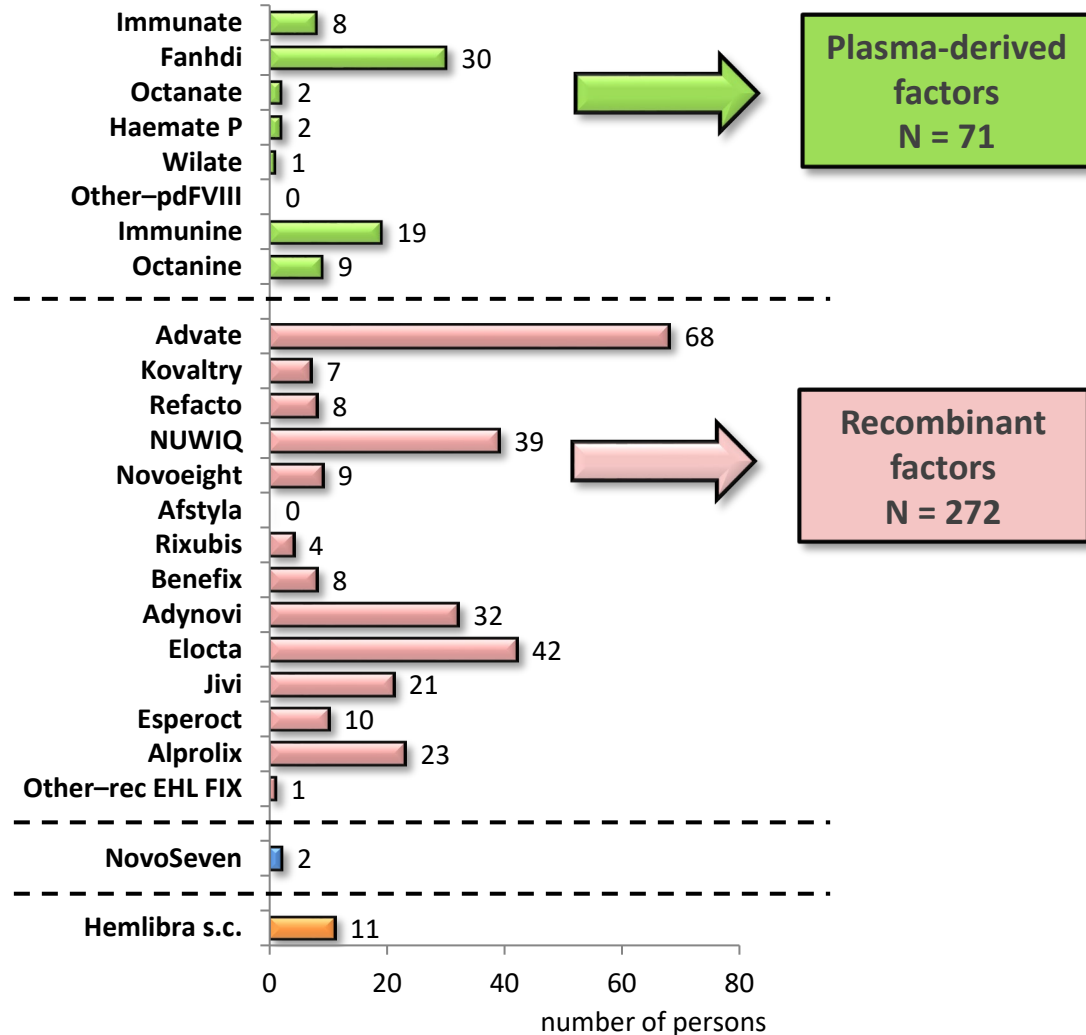
Treatment



139 children (50.2% of all PWH) were treated in 2021 (**67 children received standard factor concentrates**, 71 children EHL factors, by-pass therapy or emicizumab; 31 children received more than one type/brand of concentrate). None child were treated with both plasma-derived and recombinant factor.

¹missing type of treatment in 1 child

Treatment



283 adults (52.1% of all PWH) were treated in 2021 (**197 adults received standard factor concentrates**, 86 adults EHL factors, by-pass therapy or emicizumab; 63 adults received more than one type/brand of concentrate).
9 adults were treated with both plasma-derived and recombinant factor.

Comparison of treatment in years 2021 and 2020

	2021			2020		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	397	48.5	100.0	383	47.6	100.0
<i>Plasma-derived factor</i>	73	8.9	18.4	86	10.7	22.5
<i>Recombinant factor</i>	200	24.4	50.4	234	29.1	61.1
<i>Recombinant f. EHL</i>	200	24.4	50.4	127	15.8	33.2
<i>Emicizumab</i>	39	4.8	9.8	24	3.0	6.3
Without treatment	421	51.5	-	421	52.4	-
Total	818	100.0	-	804	100.0	-

* One patient could have more type of factor concentrates.

Comparison of treatment in years 2021 and 2020

	2021			2020		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	122	44.4	100.0	122	46.0	100.0
<i>Plasma-derived factor</i>	4	1.5	3.3	7	2.6	5.7
<i>Recombinant factor</i>	63	22.9	51.6	95	35.8	77.9
<i>Recombinant f. EHL</i>	72	26.2	59.0	45	17.0	36.9
<i>Emicizumab</i>	28	10.2	23.0	17	6.4	13.9
Without treatment	153	55.6	-	143	54.0	-
Total	275	100.0	-	265	100.0	-

* One patient could have more type of factor concentrates.

Comparison of treatment in years 2021 and 2020

	2021			2020		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All persons treated with factor concentrates*	275	50.6	100.0	261	48.4	100.0
<i>Plasma-derived factor</i>	69	12.7	25.1	79	14.7	30.3
<i>Recombinant factor</i>	137	25.2	49.8	139	25.8	53.3
<i>Recombinant f. EHL</i>	128	23.6	46.5	82	15.2	31.4
<i>Emicizumab</i>	11	2.0	4.0	7	1.3	2.7
Without treatment	268	49.4	-	278	51.6	-
Total	543	100.0	-	539	100.0	-

* One patient could have more type of factor concentrates.

Consumption of drugs

All

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person	
FVIII (IU)	<i>Immunate</i>	278 500	8	34 812.5	
	<i>Fanhdi</i>	2 072 000	30	69 066.7	
	<i>Octanate</i>	1 145 250	5	229 050.0	
	<i>Haemate P</i>	13 500	3	4 500.0	
	<i>Wilate</i>	11 000	2	5 500.0	
	<i>Other plasma-derived</i>	204	3	68.0	
	FVIII PD total	3 520 250	47	74 898.9	
	<i>Advate</i>	8 552 375	106	80 682.8	
	<i>Kovaltry</i>	1 246 000	19	65 578.9	
	<i>Refacto</i>	183 000	9	20 333.3	
	<i>NUWIQ</i>	4 128 100	42	98 288.1	
	<i>Novoeight</i>	1 733 500	11	157 590.9	
	<i>Afstyla</i>	96 500	3	32 166.7	
FVIII REC total	15 939 475	182	87 579.5		
	Standard FVIII total	19 459 725	221	88 053.1	
	<i>Adynovi</i>	9 356 654	46	203 405.5	
	<i>Elocta</i>	12 061 990	85	141 905.8	
	<i>Jivi</i>	4 079 000	26	156 884.6	
	<i>Esperoct</i>	2 777 500	12	231 458.3	
	FVIII REC EHL total	28 275 144	165	171 364.5	
	FVIII total	47 734 869	331	144 214.1	
FIX (IU)	<i>Immunine</i>	421 600	17	24 800.0	
	<i>Octanine</i>	400 000	9	44 444.4	
	FIX PD total	821 600	24	34 233.3	
	<i>Rixubis</i>	1 052 250	10	105 225.0	
	<i>Benefix</i>	914 000	8	114 250.0	
	FIX REC total	1 966 250	18	109 236.1	
		Standard FIX total	2 787 850	41	67 996.3
		<i>Alprolix</i>	2 934 450	32	91 701.6
		<i>Other-rec EHL FIX</i>	42 000	2	21 000.0
		FIX REC EHL total	2 976 450	34	87 542.6
	FIX total	5 764 300	64	90 067.2	
By-pass	<i>NovoSeven (mg)</i>	1 640.0	7	234.3	
Emicizumab	<i>Hemlibra s.c. (mg)</i>	118 454	39	3 037.3	



Consumption of drugs

Children

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	<i>Immunate</i>	0	0	
	<i>Fanhdi</i>	0	0	
	<i>Octanate</i>	703 250	3	234 416.7
	<i>Haemate P</i>	1 000	1	1 000.0
	<i>Wilate</i>	4 000	1	4 000.0
	<i>Other plasma-derived</i>	204	3	68.0
	FVIII PD total	708 250	4	177 062.5
	<i>Advate</i>	2 434 225	38	64 058.6
	<i>Kovaltry</i>	550 500	12	45 875.0
	<i>Refacto</i>	20 000	1	20 000.0
	<i>NUWIQ</i>	972 200	3	324 066.7
	<i>Novoeight</i>	499 000	2	249 500.0
	<i>Afstyla</i>	96 500	3	32 166.7
FVIII REC total	4 572 425	57	80 218.0	
Standard FVIII total	5 280 675	61	86 568.4	
<i>Adynovi</i>	2 012 000	14	143 714.3	
<i>Elocta</i>	4 164 490	43	96 848.6	
<i>Jivi</i>	451 000	5	90 200.0	
<i>Esperoct</i>	246 000	2	123 000.0	
FVIII REC EHL total	6 873 490	61	112 680.2	
FVIII total	12 154 165	106	114 661.9	
FIX (IU)	<i>Immunine</i>	0	0	
	<i>Octanine</i>	0	0	
	FIX PD total	0	0	
	<i>Rixubis</i>	392 750	6	65 458.3
	<i>Benefix</i>	0	0	
	FIX REC total	392 750	6	65 458.3
	Standard FIX total	392 750	6	65 458.3
	<i>Alprolix</i>	771 750	10	77 175.0
	<i>Other-rec EHL FIX</i>	32 000	1	32 000.0
	FIX REC EHL total	803 750	11	73 068.2
	FIX total	1 196 500	16	74 781.3
By-pass	<i>NovoSeven (mg)</i>	1 610.0	5	322.0
<i>Emicizumab</i>	<i>Hemlibra s.c. (mg)</i>	41 220	28	1 472.2

Consumption of drugs

Adults

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	<i>Immunate</i>	278 500	8	34 812.5
	<i>Fanhdi</i>	2 072 000	30	69 066.7
	<i>Octanate</i>	442 000	2	221 000.0
	<i>Haemate P</i>	12 500	2	6 250.0
	<i>Wilate</i>	7 000	1	7 000.0
	<i>Other plasma-derived</i>	0	0	
	FVIII PD total		2 812 000	43
	<i>Advate</i>	6 118 150	68	89 972.8
	<i>Kovaltry</i>	695 500	7	99 357.1
	<i>Refacto</i>	163 000	8	20 375.0
	<i>NUWIQ</i>	3 155 900	39	80 920.5
	<i>Novoeight</i>	1 234 500	9	137 166.7
	<i>Afstyla</i>	0	0	
	FVIII REC total		11 367 050	125
	Standard FVIII total	14 179 050	160	88 619.1
	<i>Adynovi</i>	7 344 654	32	229 520.4
	<i>Elocta</i>	7 897 500	42	188 035.7
	<i>Jivi</i>	3 628 000	21	172 761.9
	<i>Esperoct</i>	2 531 500	10	253 150.0
	FVIII REC EHL total	21 401 654	104	205 785.1
	FVIII total	35 580 704	225	158 136.5
FIX (IU)	<i>Immunine</i>	421 600	17	24 800.0
	<i>Octanine</i>	400 000	9	44 444.4
	FIX PD total	821 600	24	34 233.3
	<i>Rixubis</i>	659 500	4	164 875.0
	<i>Benefix</i>	914 000	8	114 250.0
	FIX REC total	1 573 500	12	131 125.0
	Standard FIX total	2 395 100	35	68 431.4
	<i>Alprolix</i>	2 162 700	22	98 304.5
	<i>Other-rec EHL FIX</i>	10 000	1	10 000.0
	FIX REC EHL total	2 172 700	23	94 465.2
	FIX total	4 567 800	48	95 162.5
By-pass	<i>NovoSeven (mg)</i>	30.0	2	15.0
	<i>Emicizumab Hemlibra s.c. (mg)</i>	77 234	11	7 021.2

Zpráva o auditech center ČNHP

Audity proběhly ve všech centrech říjen 2021- duben 2022

Závěry: certifikáty od 1.1.2022 pro všechna centra, ALE NOVĚ:

- změny v Hradci Králové

- dospělé centrum neauditováno (nyní na vlastní žádost mimo ČNHP)

- dětské centrum nově/dočasně jako Centrum přidružené péče k CCC (Praha/Brno – spádově dle preference rodiny) – tj. aktuálně nesplňuje počty pacientů pro HTC a taktéž aktuálně chybí dostatečné personální zajištění – nově: všechny nové diagnózy, zahájení/výběr léčby, plánování a zajištění výkonů, dispensární prohlídka 1 x ročně v gesci CCC. V HK i nadále aplikace faktoru, aktuální řešení krvácení aj.

- připomínka pro všechny: vhodné zajištění aktualizovaných souhlasů s domácí léčbou zohledňující SC aplikaci (Hemlibra)

- K diskuzi: frekvence auditů – viz deklarace: prodloužení certifikátu bez auditu možné max. na 2 roky, jinak mezi audity 4 roky



Informace z PedNet

- Dvě dětská centra ČNHP (Brno a Praha) se staly členy PedNet
- PedNet ocenuje kvalitu a včasnost zadávání dat z českých center
- PedNet uvažuje o rozšíření na některé další dg (probíhá dotazníková akce)
- Od roku 2020 jsou součástí dat (a tím i publikací) PedNet česká data
- FN Brno poskytla na PedNet institucionální podporu pro sběr dat



Změny v registru, otázky k datamanagementu

- Přidání nepovinné skupiny k hladině emicizumabu

AKTUÁLNÍ LÉČBA FAKTOROVÝMI/NEFAKTOROVÝMI DERIVÁTY

1

* Typ	* Typ preparátu	* Specifikace	Specifikujte jiný	* Kumulativní dávka za rok	Jednotky
Emicizumab	-	Hemlibra s.c.		6240	mg

FUNKČNÍ HLADINA EMICIZUMABU

1

Datum odběru	Aktuální hladina emicizumabu (ug/ml)

- Drobné úpravy labelů otázek, názvů skupin nebo tlačítek
- Přidání tlačítka na přenos (hmotnosti, infekcí, expozičních dnů)
- Přeskládání skupin otázek, aby na sebe navazovaly

Plán data-monitoringu na rok 2023

- **Poslední data monitoring proběhl v roce 2017(?)**
- **Proto vhodné naplánovat pro rok 2023**
- **Opět potřeba vybrat vhodné auditory (ideálně z těch, kteří zadávají data) – prosím o návrhy za jednotlivá centra do konce roku 2022 !!!**
- **Realizace ideálně v průběhu jara 2023 – tak, aby byl audit ukončen do začátku léta 2023. Detailní plán bude vytvořen systémem doodle s ohledem na možnosti auditorů**
- **Dnes prosíme o to, aby se auditoři přihlásili, bude opět honorováno**



Aktualizace deklaráce ČNHP

- Deklarace ČNHP je původní
- V principu není třeba deklarace projektů měnit
- Došlo nicméně k některým změnám, zejména v léčbě, názvu atestací, počtu CCC a podobně
- Proto navrhujeme aktualizaci (nikoli principiální změnu) deklarace
- Kolektiv Smejkal, Zápotocká, Čermáková, Blatný připravili návrh změn, který byl rozeslán předem
- Dnes bude třeba hlasovat o jeho schválení



Integrace dětských dat o ITP do registru ČNHP ve spolupráci s PSDH

– Pracovní skupina pro dětskou hematologii

- Rozšíření registru ČNHP o danou dg
- Zatím jen pro dětská centra PSDH
- Probíhá jednání s komerčními partnery stran podpory této možné budoucí části registru ČNHP
- Datamanažer stejný/jiný
- Bude procesováno odděleně od vrozených koagulopatií
- Přidání ITP neovlivní stávající sběr dat o vrozených koagulopatiích (ani finančně)
- Partnerem pro analýzy – IBA s.r.o.



Návrh změny grafiky webu ČNHP

- Web zastaralý, neintuitivní
- Připravujeme nový grafický návrh i nové programování celého webu
- Po obdržení cenové nabídky a představení návrhu budou centra oslovena (hlasování/schválení)
- Mutace i v AJ
- Cílem zjednodušení, větší viditelnost v rámci internetu, konkurenceschopnost, PR
- Větší vytěžení existujících dat
- Přátelštější prostředí
- Dnes je třeba (pouze) schválit tento záměr



Návrhy na akademické analýzy/PhD projekty nad daty ČNHP

- Prosíme centra o návrh takových projektů/analýz na rok 2023
- Bud nyní nebo do konce měsíce emailem



Všem účastníkům a hostům

Děkujeme za pozornost

