

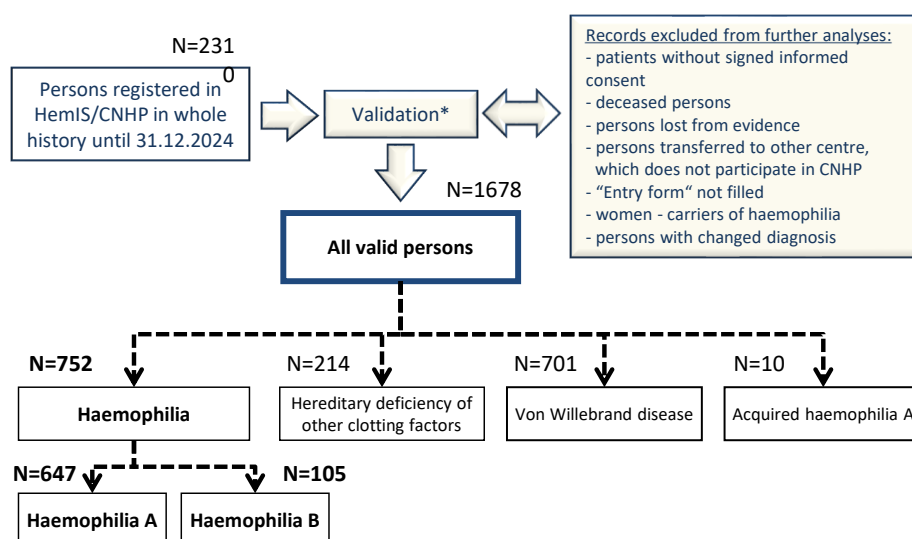


The status of care for persons with haemophilia registered within CNHP registry Annual Report 2024

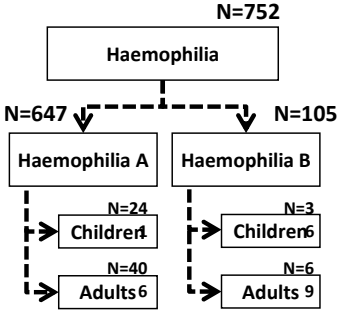
Jan Blatný, Petra Ovesná
on behalf of
Centres contributing to CNHP registry
(Czech National Haemophilia Programme)
Export date: April 9, 2025



Sample size, valid records



Persons with
haemophilia (PWH)



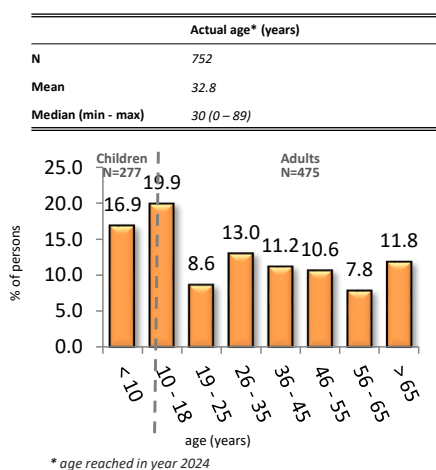
Centres participating in CNHP

Paediatric centres	Valid persons	
	N	%
Prague – Dpt. of Pediatric Haematology and Oncology, CUH Motol	110	14.6
Brno – Dpt. of Pediatric Haematology, CUH Brno	69	9.2
Hradec Králové – Dpt. of Pediatric Medicine, UH HK	31	4.1
Ostrava – Dpt. of Pediatric Medicine, UH Ostrava	19	2.5
Ústí n.L. – Pediatric Dpt. – Haematology, Masaryk Hospital	16	2.1
České Budějovice – Pediatric Dpt., Hospital CB	15	2.0
Olomouc – Dpt. of Pediatric Medicine, UH Olomouc	13	1.7
Pilsen – Pediatric Dpt., UH Pilsen	12	1.6

Adult centres	Valid persons	
	N	%
Brno – Dpt. Of Clin Hematol, UH Brno	175	23.3
Ostrava – Blood centre, UH Ostrava	75	10.0
Olomouc – Haemato-Oncology Dpt., UH Olomouc	58	7.7
Pilsen – Dpt. of Biochemistry and Hematology, UH Pilsen	53	7.0
Liberec – Dpt. Of Clin Hematol, Hospital Liberec	48	6.4
Ústí n.L. – Dpt. Of Clin Hematol, Masaryk Hospital	29	3.9
České Budějovice – Dpt. Of Clin Hematol, Hospital CB	25	3.3
Nymburk – Center for Thrombosis and Hemostasis, Nymburk Hospital	4	0.5

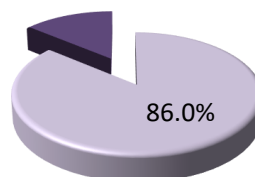
Basic demographics

All
N=752



Type of haemophilia

Haemophilia A
(N=647)
Haemophilia B
(N=105)



Four children with haemophilia were born in 2024.

Persons with haemophilia and inhibitors in 2024

All
N=752

Active inhibitors were recorded in 12 persons during 2024

- 1 inhibitor in adult with mild HA newly developed and one child had re-occurrence in 2024

PWH with inhibitors:

- 6 children and 6 adults
- 11 haemophilia A and 1 haemophilia B
- 9 in severe, 2 in moderate and 1 in mild haemophilia
- 11 high-titre and 1 low-titre (<5BU)
- 7 high response and 4 low response inhibitors; this information not available in 1 PWH with inhibitor
- 10 patients were treated with emicizumab
 - 7 patients were treated only with emi, 1 patient 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
- One patient has already been on-going ITT in 2024 (started earlier)

Eradication of inhibitor:

- Six patients with a history of inhibitor (now receiving emicizumab prophylaxis) had repeatedly negative inhibitor levels and were considered inhibitor-free this year
- One patient had transient inhibitor (increased inhibitor level in 2023) and was inhibitor-free in 2024
- One patient with inhibitor was transferred to another center (outside CNHP registry)

Summary description of the PWH with inhibitors within registry. There are around five other PWH with inhibitors in the centre not participating in CNHP registry.

Number of PWHI further decreased in 2024. Only 1 new inhibitors developed (adult with mild HA receiving FVIII before surgery) and spontaneously disappeared without any treatment and reoccurred in 1 child with previous history of inhibitors after FVIII treatment for bleed.

The reason for the decrease of PWHs with inhibitors is caused mainly by disappearing of inhibitors in those on long-term prophylaxis with emi

ABR and treatment regimens in patients with inhibitor

N=12

	Type	Year of birth	Severity	ITT	Emi	By-pass	Titre	Responder	ABR	Joint / other
1	HA	2021	Severe		Yes	OD	high	HR	3	1 / 2
2	HA	2020	Severe	Yes	Yes		high	NA	3	1 / 2
3	HA	2016	Severe		Yes		high	LR	2	1 / 1
4	HA	2015	Severe		Yes		high	HR	0	0 / 0
5	HA	1977	Severe		Yes		high	HR	0	0 / 0
6	HA	1975	Severe		Yes		high	HR	0	0 / 0
7	HA	1971	Severe		Yes		high	LR	0	0 / 0
8	HA	1956	Severe		Yes		high	LR	0	0 / 0
9	HA	2011	Moderate		Yes		high	HR	0	0 / 0
10	HA	1941	Moderate		Yes		high	HR	0	0 / 0
11	HA	1988	Mild		No		low	LR	1	1 / 0
12	HB	2007	Severe			Permanent px	high	HR	15	11 / 4

new in 2024
NA not available

This slide describes in more details all PWH with “active” inhibitors within CNHP registry. Very little patients with HT inhibitors remained on ITI (only one in fact, being on concomitant emi prophaxis). All HA hi gh titre inhibitor patients were started or switched on/to emicizumab. The number of bleeds, on above mentioned treatment is kept low.

ABR according to treatment regimen in PWH with inhibitor

N=12

Diagnosis	ITT	Emi/by-pass prophylaxis	N	ABR (mean)	ABR (median, min-max)	Joint / other bleeds (median)
Haemophilia A	Yes	Emi px	1	3.00	3 (3-3)	1 / 2
		OD	0	-	-	-
	No	Emi px	9	0.56	0 (0-3)	0 / 0
		OD	1	1.00	1 (1-1)	1 / 0
Haemophilia B	No	BPA permanent	1	15.00	15 (15-15)	11 / 4

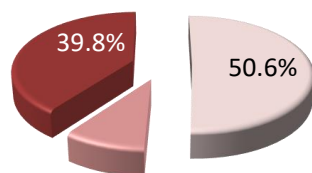
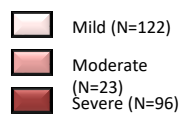


Demographic characteristics Haemophilia A

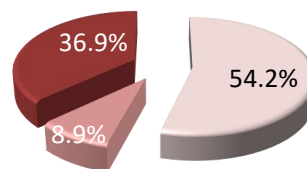
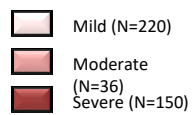
Severity of haemophilia A

Haem A
N=647

Children (N=241)

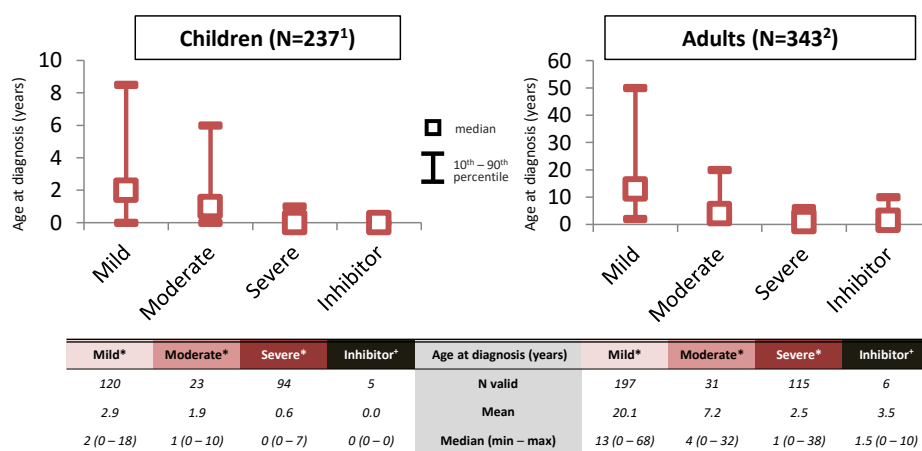


Adults (N=406)



Age at diagnosis according to severity of haemophilia A

Haem A
N=647



¹ Missing information on year of diagnosis in 4 children.

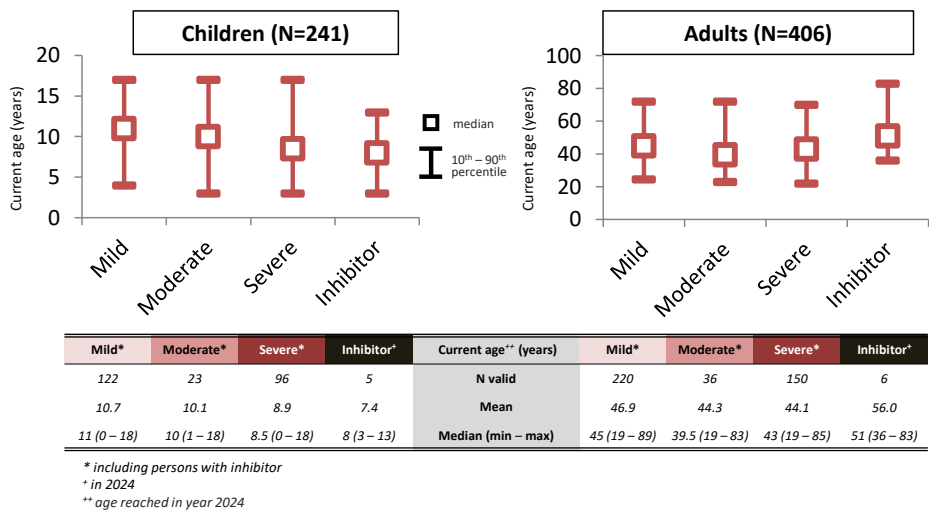
² Missing information on year of diagnosis in 63 adults.

* Including persons with inhibitor
* in 2024

Actual age according to severity of haemophilia

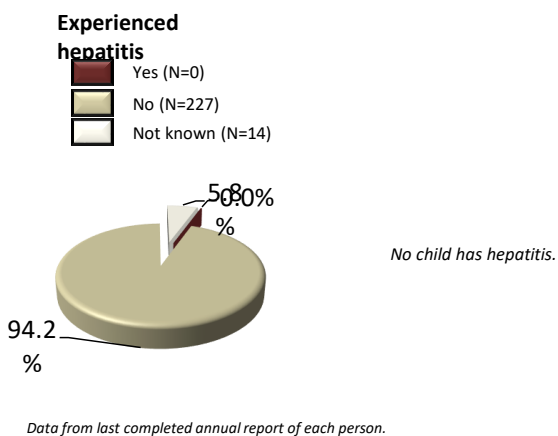
A

Haem A
N=647



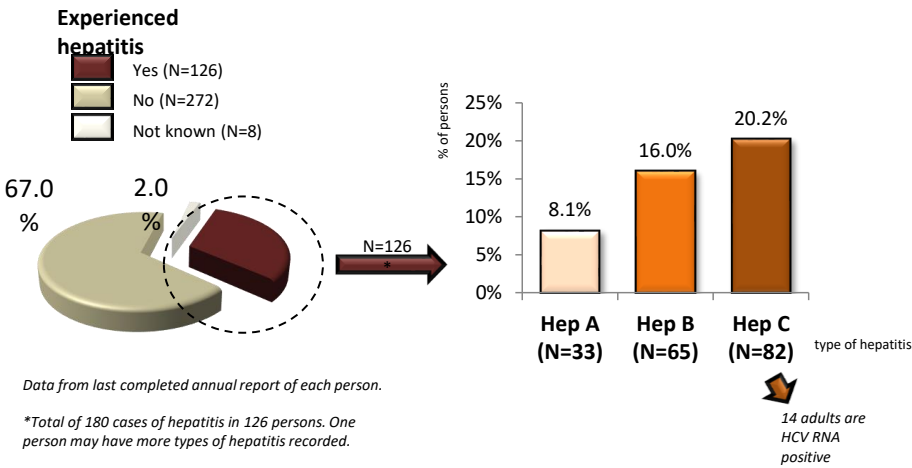
Hepatitis (ever) experienced

Children
with
Haem A
N=241



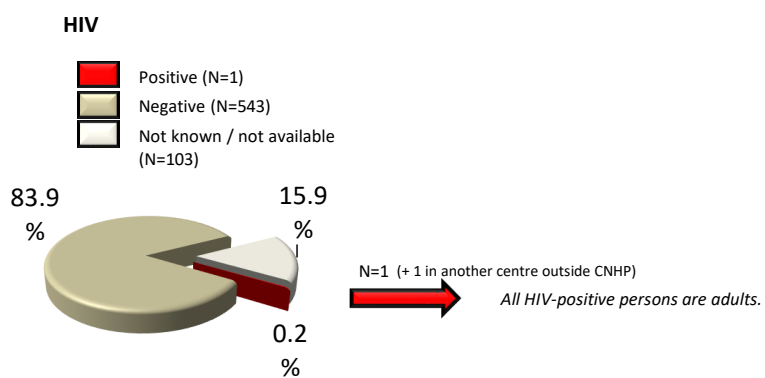
Hepatitis (ever) experienced

Adults
Haem A
N=406



HIV

All
Haem A
N=647



Data from last completed annual report of each person.



Treatment outcomes and bleeding frequency Haemophilia A

Data from year 2024 – sample size

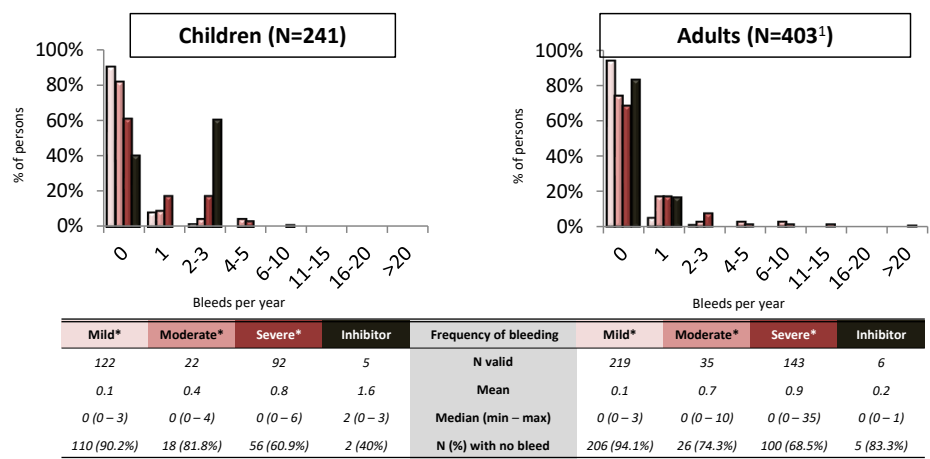
All
Haem A
N=647

	Valid persons			Persons with valid annual report			Persons examined			Persons treated	
	N	%		N	%		N	%		N	%
All	647	100%	→	627	96.9%	→	479	74.0%	→	336	51.9%
of them with inhibitor	11			11			11			11	
Children	241	100%	→	241	100.0%	→	211	87.6%	→	126	52.3%
of them with inhibitor	5			5			5			5	
Adults	406	100%	→	386	95.1%	→	268	66.0%	→	210	51.7%
of them with inhibitor	6			6			6			6	

There are records of nearly 75% of all Czech haemophiliacs in total within the CNHP registry in 2024. As for paediatric population, ALL children are recorded. CNHP registry also houses records of about 65% of adult haemophiliacs in Czech Republic. Further slides display analyses performed only on records, which were updated during 2024. Not all patients came to the centre (especially adults) and not all centres fully reported all data (including detailed info about bleeding episodes) in 2024. Thus, not all records have been updated and used for further analyses. Data monitoring has been introduced since 2017 to further increase the validity of the data within CNHP registry. Last data monitoring by the teams of auditors was done in all centres in 2023.

Frequency of bleeding requiring treatment in 2024

Haem A
N=647



* without inhibitor

¹ Frequency of bleeding is missing in 3 adults.

Data shown support good efficacy of care provided to Czech PWH, no matter what age category they are. Mean/Median number of bleedings per year (ABR) is 0.8/0 for children and 10.9/0 for adults with severe haemophilia A without inhibitors. WAS 0.9/0 for children and 1.2/0 for adults in 2023.

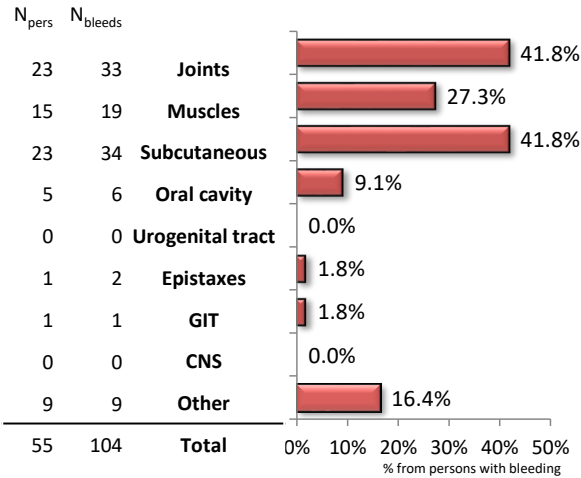
The median of ABR is kept 0 for children and adults in CZ since 2021. This year, median ABR was 0 also for adult PWHs with inhibitors.

Location of bleeds in 2024

Children
Haem A
N=241

55 (22.8%) children experienced bleeding at least once in year; 104 bleeds were recorded in total, 6 bleeds required hospitalization. All 55 children have recorded location of their bleeds.

186 (77.2%) children recorded no bleed during year 2024.



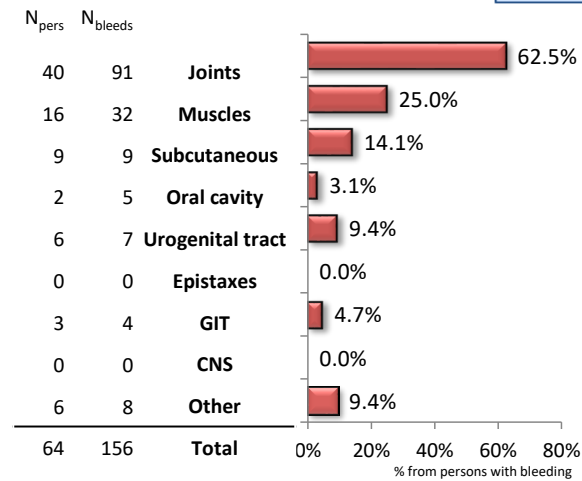
Location of bleeds in 2024

Adults
Haem
A
N=403
1

66 (16.4%) adults experienced bleeding at least once in year; 174 bleeds were recorded in total, 15 bleeds required hospitalization. 64 of these 66 adults have recorded location of their bleeds. Localization is not known in 2 adults.

337 (83.6%) adults have recorded no bleed during year 2024.

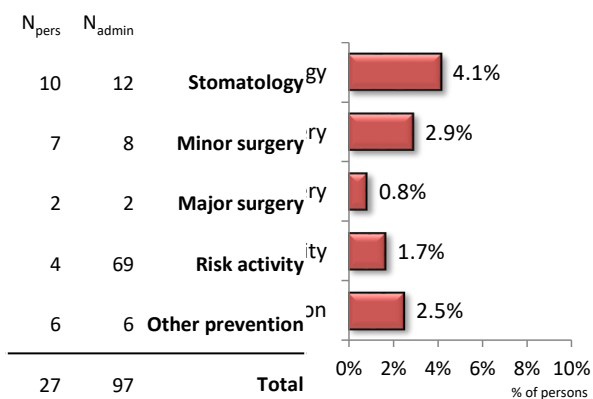
¹Frequency of bleeding is missing in 3 adult.



Preventive administration in 2024

Children
with
Haem A
N=241

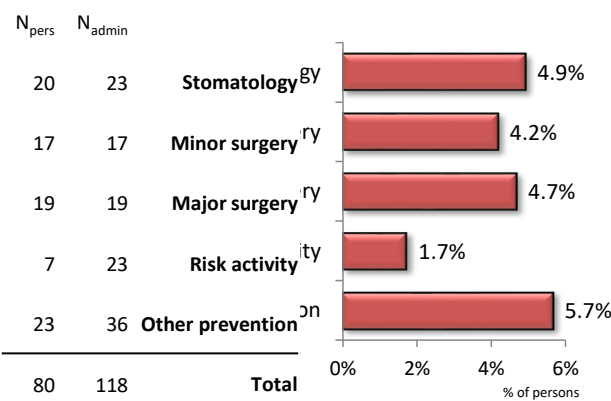
27 (11.2%) children were given factor to prevent bleeding during/before risk situation. 97 preventive administrations were recorded in total.



Preventive administration in 2024

Adults
Haemophilia A
N=406

80 (19.7%) persons were given factor to prevent bleeding during/before risk situation.
118 preventive administrations were recorded in total.



This figure refers to preventive treatment in adults with HA. No significant change compared to 2023



ABR according to treatment regimen Haemophilia A without inhibitor

Annual bleeding rate according to treatment regimen

Children
Haem A
N=236*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	121	1	13	9	2	90
Mean	0.1	0.0	0.3	0.4	0.5	0.8
Median (min – max)	0 (0 – 3)	0 (0 – 0)	0 (0 – 4)	0 (0 – 2)	0.5 (0 – 1)	0 (0 – 6)
Total no of recorded bleeds	15	0	4	4	1	72
Children on permanent prophylaxis	1 (0.8%)		9 (40.9%)		90 (97.8%)	
% of factor (FVIII) consumed by children on permanent prophylaxis	0.4%		96.2%		95.7%	
Location of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	121	1	13	9	2	90
JOINT BLEEDS						
Mean	0.0	0	0.1	0.2	0.0	0.3
Median (range)	0 (0 – 2)	0 (0 – 0)	0 (0 – 1)	0 (0 – 1)	0 (0 – 0)	0 (0 – 4)
Total no of recorded bleeds	2	0	1	2	0	25
OTHER BLEEDS						
Mean	0.1	0	0.2	0.2	0.5	0.5
Median (range)	0 (0 – 3)	0 (0 – 0)	0 (0 – 3)	0 (0 – 2)	0.5 (0 – 1)	0 (0 – 6)
Total no of recorded bleeds	13	0	3	2	1	47

* without inhibitor

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

This slide confirms good effect of permanent prophylaxis in children. Rate of prophylaxis (factor or emi) is almost 100% among children with severe haemophilia A. Number of bleeds per year (median) in severe haemophiliacs A on prophylaxis is kept on 0.

It is true also for joint bleeds, see the lower figure on the slide. The total number of joint bleeds decrease almost 5 times compared to 2023!!!

Annual bleeding rate according to treatment regimen

Adults
Haem A
N=397*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	219	0	27	8	26	117
Mean	0.1	0.0	0.6	1.0	2.9	0.5
Median (min – max)	0 (0 – 3)	(–)	0 (0 – 10)	0.5 (0 – 5)	0 (0 – 35)	0 (0 – 7)
Total no of recorded bleeds	16	0	16	8	76	57
Adults on permanent prophylaxis	0 (0%)		8 (22.9%)		119 (81.5%)	
% of factor (FVIII) consumed by adults on permanent prophylaxis	0.0%		85.3%		91.2%	
Location of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	219	0	27	8	24	117
JOINT BLEEDS						
Mean	0.0	0	0.1	0.8	1.7	0.3
Median (range)	0 (0 – 1)	(–)	0 (0 – 3)	0 (0 – 4)	0 (0 – 25)	0 (0 – 4)
Total no of recorded bleeds	6	0	4	6	41	33
OTHER BLEEDS						
Mean	0.0	0	0.4	0.3	0.7	0.2
Median (range)	0 (0 – 3)	(–)	0 (0 – 9)	0 (0 – 1)	0 (0 – 10)	0 (0 – 4)
Total no of recorded bleeds	10	0	12	2	17	24

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

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

Prophylaxis works very well in Czech adult PWHs! It keeps the median of ABR in all severe haemophiliacs with prophylaxis below 1. Was 0 in 2023, though ?!
Rate of prophylaxis (factor or emi) is kept around 92% among adults with severe haemophilia A.

Median of joint bleeds per year is 0 in adults with severe HA (both on prophylaxis and OD)! It is however seen, that some adult PWHA still have significant number of joint bleeds despite the prophylaxis.

ABR according to treatment regimen and age

Adults
Haem A
N=397*

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

missing location

Frequency of bleeding	Mild*		Moderate*		Severe*		Adults (haem A) born before 1990 N=269
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	
N valid	153	0	16	6	20	74	
Mean	0.1	0.0	0.1	1.2	3.7	0.5	
Median (min – max)	0 (0 – 3)	(–)	0 (0 – 1)	0.5 (0 – 5)	0 (0 – 35)	0 (0 – 5)	
Total no of recorded bleeds	12	0	1	7	74	35	
Adults on permanent prophylaxis	0 (0%)		6 (27.3%)		76 (78.4%)		
% of factor (FVIII) consumed by adults on permanent prophylaxis	0.0%		83.7%		89.8%		
Frequency of bleeding	Mild*		Moderate*		Severe*		Adults (haem A) born in 1990 or later N=128
Treatment regimen	OD	Prophy	OD	Prophy	OD	Prophy	
N valid	66	0	11	2	6	43	
Mean	0.1	0.0	1.4	0.5	0.3	0.5	
Median (min – max)	0 (0 – 2)	(–)	0 (0 – 10)	0.5 (0 – 1)	0 (0 – 1)	0 (0 – 7)	
Total no of recorded bleeds	4	0	15	1	2	22	
Adults on permanent prophylaxis	0 (0%)		2 (15.4%)		43 (87.8%)		
% of factor (FVIII) consumed by adults on permanent prophylaxis	0.0%		87.9%		93.9%		

This important table shows in general difference in bleeding rates between adult PWH born before 1990 (when concentrates and thus also prophylaxis became available in CZ) and PWH and born later. This is now however true only for means and intervals. Median of ABR in severe HA on prophylaxis is below 1 for both groups.

Prophylaxis thus works very well also in most of those, with already damaged joints. This is clearly seen on median ABR for the “older” group. We thus further advocate for more tertiary prophylaxis in adult PWH.

Joint and other bleeds according to treatment regimen and age

Adults
Haem A
N=395*

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

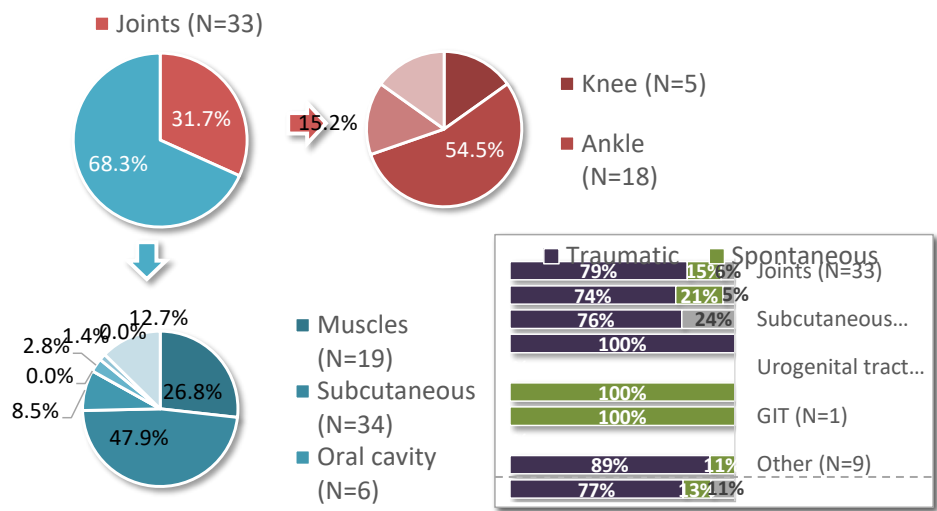
Frequency of bleeding		Mild*		Moderate*		Severe*		Adults (haem A) born before 1990 N=267
Treatment regimen		OD	prophy	OD	prophy	OD	prophy	
N valid		153	0	16	6	18	74	
JOINT BLEEDS								
Mean		0.0	0	0.0	0.8	2.2	0.3	
Median (range)		0 (0 – 1)	(–)	0 (0 – 0)	0 (0 – 4)	0 (0 – 25)	0 (0 – 4)	
Total no of recorded bleeds		3	0	0	5	40	25	
OTHER BLEEDS								
Mean		0.1	0	0.1	0.3	0.9	0.1	
Median (range)		0 (0 – 3)	(–)	0 (0 – 1)	0 (0 – 1)	0 (0 – 10)	0 (0 – 3)	
Total no of recorded bleeds		9	0	1	2	16	10	

Frequency of bleeding		Mild*		Moderate*		Severe*		Adults (haem A) born in 1990 or later N=128
Treatment regimen		OD	prophy	OD	prophy	OD	prophy	
N valid		66	0	11	2	6	43	
JOINT BLEEDS								
Mean		0.0	0	0.4	0.5	0.2	0.2	
Median (range)		0 (0 – 1)	(–)	0 (0 – 3)	0.5 (0 – 1)	0 (0 – 1)	0 (0 – 3)	
Total no of recorded bleeds		3	0	4	1	1	8	
OTHER BLEEDS								
Mean		0.0	0	1.0	0.0	0.2	0.3	
Median (range)		0 (0 – 1)	(–)	0 (0 – 9)	0 (0 – 0)	0 (0 – 1)	0 (0 – 4)	
Total no of recorded bleeds		1	0	11	0	1	14	

Location and etiology of bleeds

Children
Haem A
N=104*

* number of bleeds



Detailed treatment of bleeds

Children
Haem A
N=104*

* number of bleeds

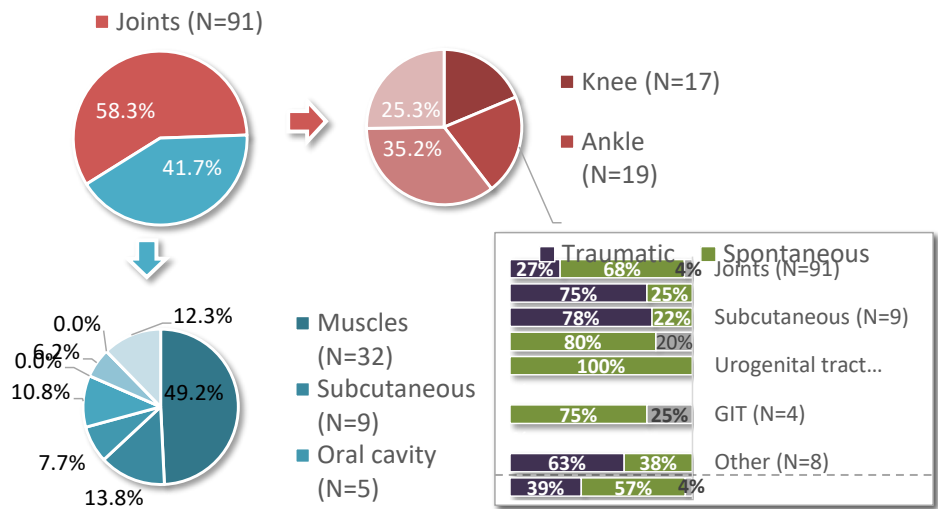
	Joints	Muscles	Subcuta- neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	33	19	34	6	0	2	1	0	9	104
FVIII consumption per bleed (IU), valid N	27	19	28	5		1	1		8	89
geometric mean	2497.3	2545.2	1359.9	574.3		1500.0	10500.0		1990.2	1887.9
median	2500.0	2500.0	1500.0	500.0		1500.0	10500.0		2250.0	1500.0
min – max	500–20000	500–19000	500–8000	250–4000		1500–1500	10500–10500		500–6250	250–20000
sum	97 200	68 750	47 750	5 500		1 500	10 500		20 250	251 450
No. of doses per bleed										
geometric mean	1.5	1.4	1.2	1.0		1.0	7.0		1.6	1.3
median	1	1	1	1		1	7		1	1
min – max	0–8	1–10	0–4	1–1		0–1	7–7		0–9	0–10
Duration of therapy per bleed, days										
geometric mean	2.0	1.7	2.5	2.7		4.6	5.0		1.7	2.1
median	1	1	1	3		5	5		2	1
min – max	1–21	1–7	1–1500	1–12		3–7	5–5		1–7	1–1500
N (%) with hospitalization	2 (6.1%)	1 (5.3%)	0 (0%)	1 (16.7%)		0 (0%)	1 (100%)		1 (11.1%)	6 (5.8%)
N (%) with rebleeding	5 (15.2%)	0 (0%)	1 (2.9%)	0 (0%)		0 (0%)	0 (0%)		1 (11.1%)	7 (6.7%)

In median, the dose used to treat joint bleed in children was 2500 IU FVIII administered in 1 infusion within one day. Muscle bleeds were often treated similarly .
Some bleeds were however cased only on prophylaxis with no extra dose needed. Compared to 2023, bleeds in 2024 needed less factor and less time to heal.

Location and etiology of bleeds

Adults
Haem A
N=156*

* number of bleeds



The situation is different in adults, though. Over 58% of bleeds in adults with haemophilia are joint bleeds, though their total number significantly decreased compared to 2023. Soft tissue bleeds are in 32% muscle bleeds. The most of all bleeds in adults are spontaneous ones (i.e. 68% of joint bleeds). This is probably mainly due to already damaged joints especially in people born before 1990.

Detailed treatment of bleeds

Adults
Haem A
N=156*

* number of bleeds

	Joints	Muscles	Subcutaneous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	91	32	9	5	7	0	4	0	8	156
FVIII consumption per bleed (IU), valid N	87	31	9	5	6		4		7	149
geometric mean	2862.9	2876.9	4313.2	1918.6	2167.5		8795.6		8572.4	3110.0
median	3000.0	1000.0	4000.0	2000.0	4000.0		16750.0		9000.0	3000.0
min – max	500–91000	1000–86000	1000–26000	1000–6500	10–24000		2000–47500		1000–63000	10–91000
sum	502 500	304 000	62 500	12 500	44 000		83 000		150 000	1 158 510
No. of doses per bleed										
geometric mean	1.7	1.9	1.7	1.3	2.8		4.8		4.3	1.9
median	1	1	1	1	3		9		5	1
min – max	0–37	0–34	1–13	1–2	0–7		1–32		1–21	0–37
Duration of therapy per bleed, days										
geometric mean	1.7	2.0	1.9	1.3	2.7		25.6		4.0	2.0
median	1	1	1	1	4		15		5	1
min – max	1–30	1–45	1–13	1–2	1–6		1–2000		1–21	1–2000
N (%) with hospitalization	5 (5.5%)	4 (12.5%)	1 (11.1%)	0 (0%)	1 (14.3%)		2 (50%)		2 (25%)	15 (9.6%)
N (%) with rebleeding	4 (4.4%)	1 (3.1%)	0 (0%)	0 (0%)	1 (14.3%)		0 (0%)		0 (0%)	6 (3.8%)

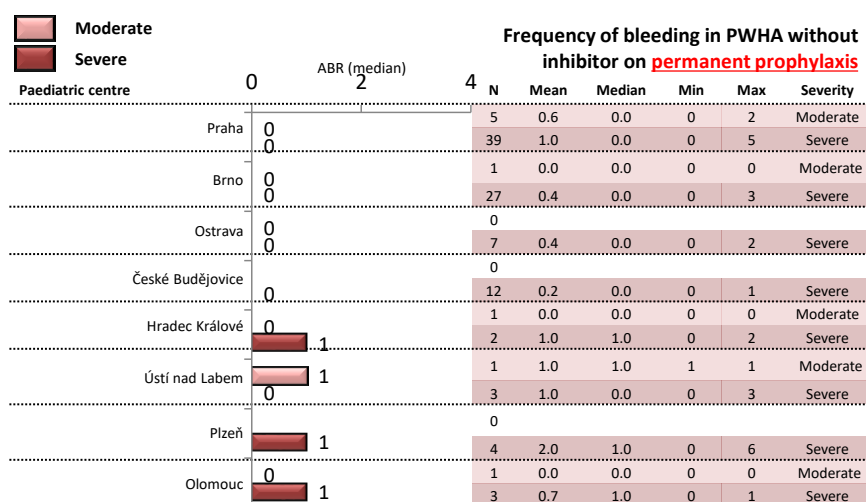
In median, the dose used to treat joint bleed in adults increased to 3000 IU FVIII administered in one infusion within one day. Muscle bleeds were often treated with total dose of 1000 IU FVIII administered in 1 infusions. Doses are similar or lower than in children, though the weight of adults is approximately 3x higher, compared to children. Some adults still received ONLY 500 IU to treat the joint/muscle bleed!!!



ABR according to centres Haemophilia A (PWHa)

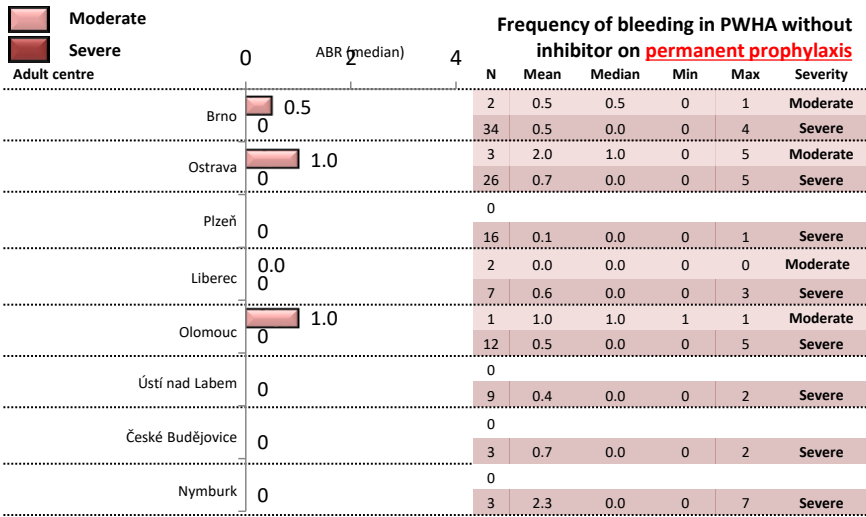
Annual bleeding rate on permanent prophylaxis

HaemA on
prophy
Paed. centres
N=106



Annual bleeding rate on permanent prophylaxis

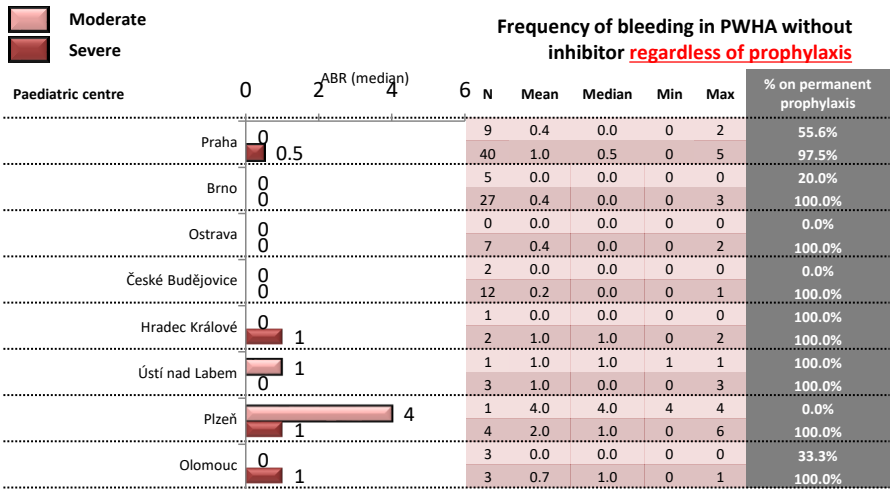
HaemA on prophylaxis
Adult centres
N=118



In 2024 the difference in ABR of adults with severe HA on prophylaxis is low between centres. All adult PWHs with severe disease on permanent prophylaxis had median of ABR below 1 in all centres for the first time. (Was 0 though in 2023?)

Annual bleeding rate regardless prophylaxis

HaemA
Paed.
centres
N=120



Ideally, children on prophylaxis should have same (lower) bleeding rate as (than) those, who do not need prophylaxis. This is in fact the goal of prophylaxis! Those, who bleed, should be on prophylaxis to decrease the bleeding rate. Those, who have not more than one joint bleed per year without prophylaxis probably do not need it. Paediatric centres should work further on this issue to reflect the fact, that children in these days want to live very active life. The discrepancy between centres should be further minimized or should ideally disappear to guarantee the same level of care nation-wide.

Annual bleeding rate regardless prophylaxis

HaemA
Adult
centres
N=172*
* missing ABR in 3
adults

Adult centre	ABR (median)				N	Frequency of bleeding in PWH without inhibitor regardless of prophylaxis				% on permanent prophylaxis
	0	2	4	6		Mean	Median	Min	Max	
Brno	0.0				15	0.2	0.0	0	1	13.3%
	0.0				42	0.4	0.0	0	4	81.0%
Ostrava	0.0				7	0.9	0.0	0	5	42.9%
	0.0				29	0.7	0.0	0	5	89.7%
Plzeň	0.0				3	0.0	0.0	0	0	0.0%
	0.0				20	1.0	0.0	0	12	80.0%
Liberec	0.0				3	0.0	0.0	0	0	66.7%
	0.0				10	2.0	0.0	0	15	70.0%
Olomouc	0.0	2.0			2	2.0	2.0	1	3	50.0%
	0.0				14	2.9	0.0	0	35	82.4%
Ústí nad Labem	0.0				3	0.0	0.0	0	0	0.0%
	0.0				10	0.4	0.0	0	2	90.0%
České Budějovice	0.0			5.0	2	5.0	5.0	0	10	0.0%
	0.0				9	0.7	0.0	0	2	33.3%
Nymburk	0.0				0					
	0.0				3	2.3	0.0	0	7	100.0%

Prophylactic regimens and treatment outcomes

HaemA
Paed.
centres
N=120

Paediatric centre	Severity	Total N	% of patients	N	PERMANENT PROPHYLAXIS								ON-DEMAND / TEMPORARY PROPHY		
					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	9	55.6%	5	82.8	82.8	64.1	68.0			0.6	0.0	4	0.3	0.0
	Severe	40	97.5%	39	70.0	88.5	72.8	70.7	1.7	1.5	1.0	0.0	1	1.0	1.0
Brno	Moderate	5	20.0%	1					1.3	1.3	0.0	0.0	4	0.0	0.0
	Severe	27	100.0%	27			83.6	87.5	1.3	1.2	0.4	0.0	0		
Ostrava	Moderate	0	0.0%	0									0		
	Severe	7	100.0%	7					1.5	1.5	0.4	0.0	0		
Č. Budějovice	Moderate	2	0.0%	0									2	0.0	0.0
	Severe	12	100.0%	12			74.3	73.6	1.4	1.4	0.2	0.0	0		
Hradec Králové	Moderate	1	100.0%	1			61.4	61.4			0.0	0.0	0		
	Severe	2	100.0%	2			91.5	91.5			1.0	1.0	0		
Ústí nad Labem	Moderate	1	100.0%	1					2.2	2.2	1.0	1.0	0		
	Severe	3	100.0%	3			58.6	58.6	1.3	1.3	1.0	0.0	0		
Plzeň	Moderate	1	0.0%	0									1	4.0	4.0
	Severe	4	100.0%	4			160.7	160.7	1.4	1.5	2.0	1.0	0		
Olomouc	Moderate	3	33.3%	1					1.2	1.2	0.0	0.0	2	0.0	0.0
	Severe	3	100.0%	3					1.5	1.5	0.7	1.0	0		

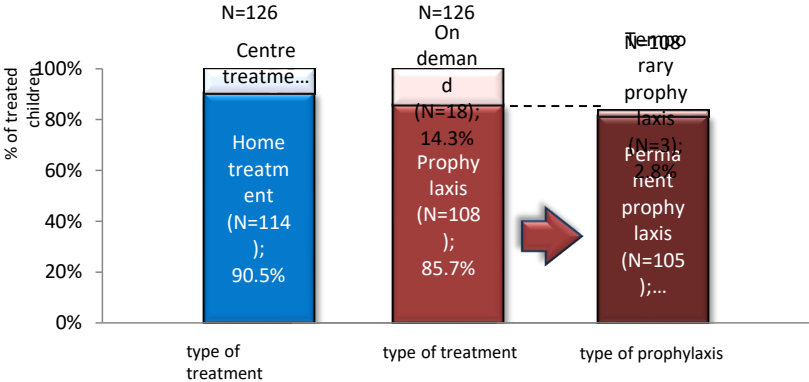
Prophylactic regimens and treatment outcomes

HaemA
Adult
centres
N=175

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			Mean	Median	Median
Brno	Moderate	15	13.3%	2			56.3	56.3	1.9	1.9	0.5	0.5	55	13	0.2	0.0	40
	Severe	42	81.0%	34	92.5	90.2	75.6	72.9	1.4	1.4	0.5	0.0	43	8	0.1	0.0	42
Ostrava	Moderate	7	42.9%	3			72.1	64.5			2.0	1.0	72	4	0.0	0.0	32
	Severe	29	89.7%	26	79.6	76.2	77.2	77.6	1.5	1.5	0.7	0.0	38	3	0.7	0.0	61
Pízeň	Moderate	3	0.0%	0										3	0.0	0.0	48
	Severe	20	80.0%	16	52.5	52.5	65.6	59.1	1.4	1.4	0.1	0.0	46	4	4.5	3.0	60
Liberec	Moderate	3	66.7%	2					1.7	1.7	0.0	0.0	51	1	0.0	0.0	43
	Severe	10	70.0%	7			76.4	73.9	1.5	1.5	0.6	0.0	40	3	5.3	1.0	25
Olomouc	Moderate	2	50.0%	1	73.2	73.2	72.5	72.5			1.0	1.0	25	1	3.0	3.0	29
	Severe	17	82.4%	14			55.0	52.2	2.2	2.2	0.5	0.0	37	3	17.5	17.5	64
Ústí n. Labem	Moderate	3	0.0%	0										3	0.0	0.0	27
	Severe	10	90.0%	9			37.9	42.9			0.4	0.0	45	1	0.0	0.0	40
Č. Budějovice	Moderate	2	0.0%	0										2	5.0	5.0	56
	Severe	9	33.3%	3			70.5	57.1			0.7	0.0	56	6	0.7	0.0	62
Nymburk	Moderate	0	0.0%	0										0			
	Severe	3	100.0%	3			81.5	81.5	1.3	1.3	2.3	0.0	34	0			

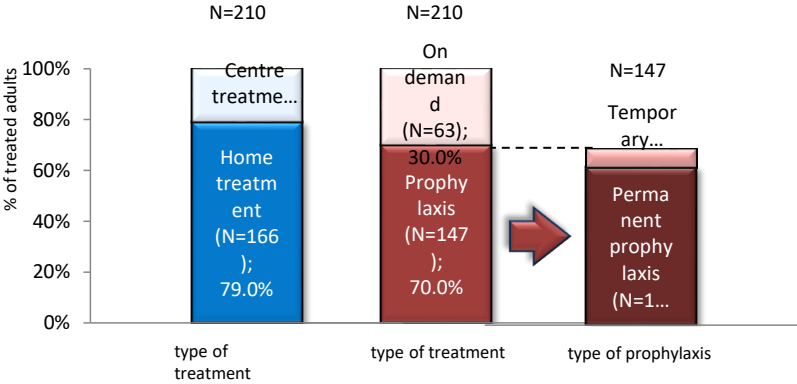
Type of treatment (subgroup of treated patients)

Children
on
Haem A
N=126



Type of treatment (subgroup of treated patients)

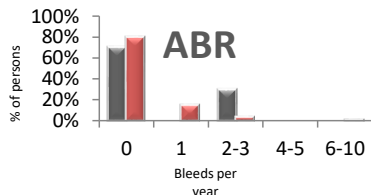
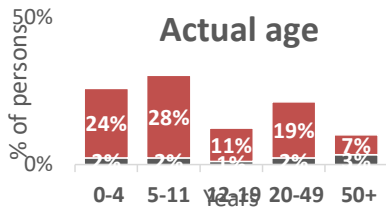
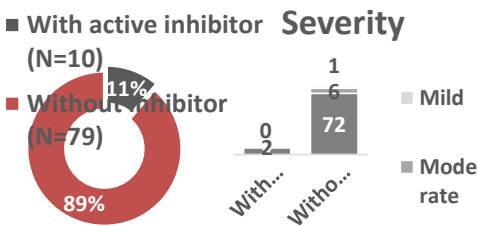
Adults
Haem
A
N=210



Emicizumab treatment in 2024

Haem A
N=89¹

¹ patients on
emicizumab prophylaxis
in a given year



	Total	With inhibitor	Without inhibitor
N valid	89	10	79
ABR			
Mean	0.36	0.80	0.30
Median (min – max)	0 (0 – 6)	0 (0 – 3)	0 (0 – 6)
N (%) with no bleed	70 (79%)	7 (70%)	63 (80%)
EMI DOSE (mg/kg/week)			
Mean	1.52	1.68	1.49
Median (min – max)	1.5 (0.8 – 4)	1.5 (1.4 – 2.9)	1.5 (0.8 – 4)
EMI FREQUENCY			
Once a week	7 (7.9%)	5 (50%)	2 (2.5%)
Once every 2 weeks	76 (85.4%)	5 (50%)	71 (89.9%)
Once a month	1 (1.1%)	0 (0%)	1 (1.3%)
Other, NA	5 (5.6%)	0 (0%)	5 (6.3%)

This slide describe the population of HA patients treated with emicizumab in CZ. The total number of PWHs on emi increased from 76 in 2023 to 89 in 2024. It is seen, that in 2024 emi was used mainly for people with HA (currently) without inhibitors and its efficacy was very high for both children and adults.



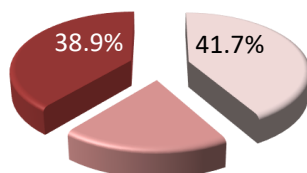
Demographic characteristics Haemophilia B

Severity of haemophilia B

Haem B
N=105

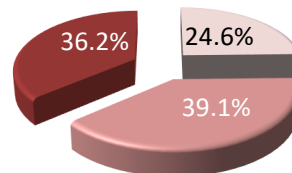
Children (N=36)

Mild (N=15)
Moderate (N=7)
Severe (N=14)



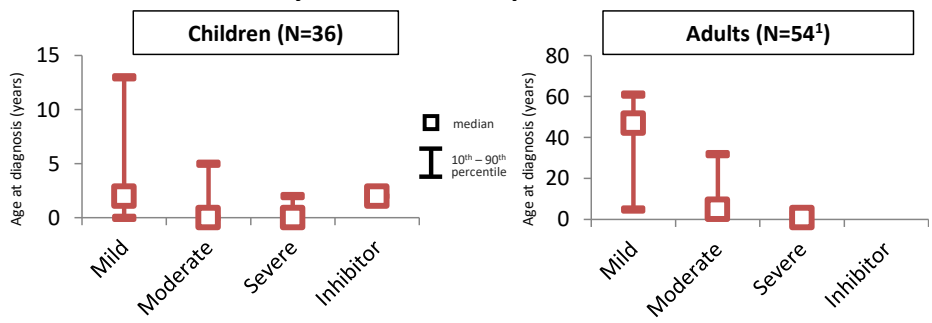
Adults (N=69)

Mild (N=17)
Moderate (N=27)
Severe (N=25)



Age at diagnosis according to severity of haemophilia B

Haem B
N=105



Mild*	Moderate*	Severe*	Inhibitor*	Age at diagnosis (years)	Mild*	Moderate*	Severe*	Inhibitor*
15	7	14	1	N valid	11	22	21	0
4.3	0.9	0.6	2.0	Mean	34.1	11.9	1.3	
2 (0–16)	0 (0–5)	0 (0–3)	2 (2–2)	Median (min–max)	47 (2–67)	5 (0–69)	1 (0–4)	

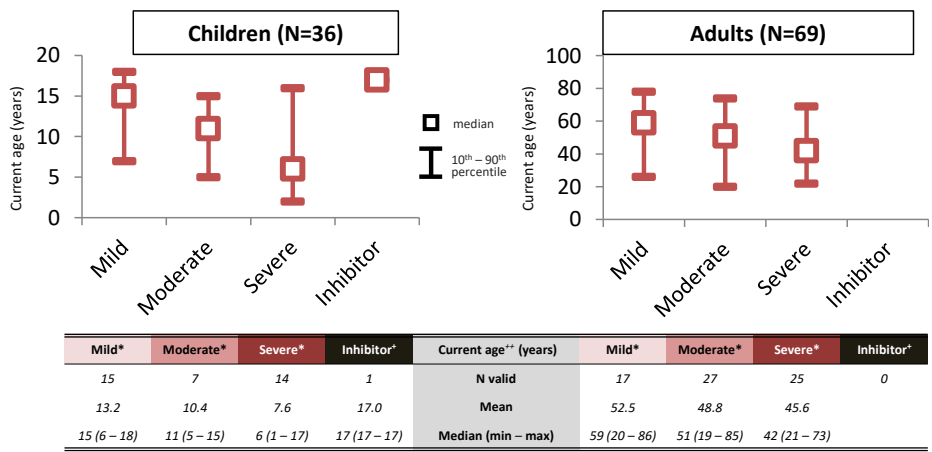
¹ Missing information on year of diagnosis in 15 adults. * including persons with inhibitor in 2024

There is no major difference in demographics between HA and HB.

Actual age according to severity of haemophilia

B

Haem B
N=105

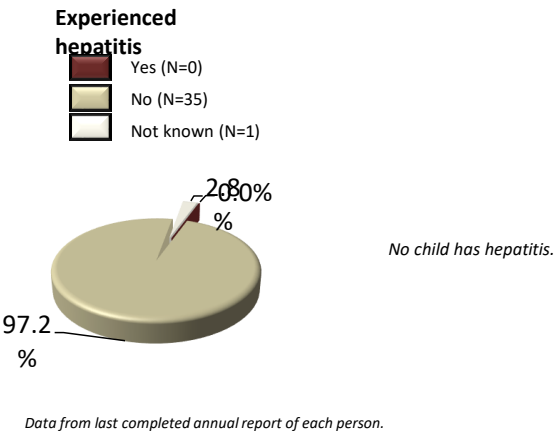


* including persons with inhibitor
* in 2024
** age reached in year 2024

There is no major difference in demographics between HA and HB, perhaps adults with HB are slightly older than those, with HA.

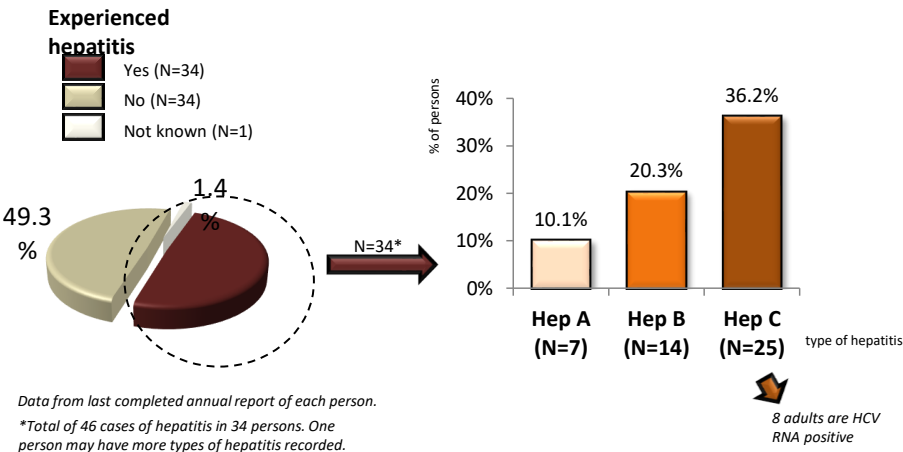
Hepatitis (ever) experienced

Children
with
Haem B
N=36



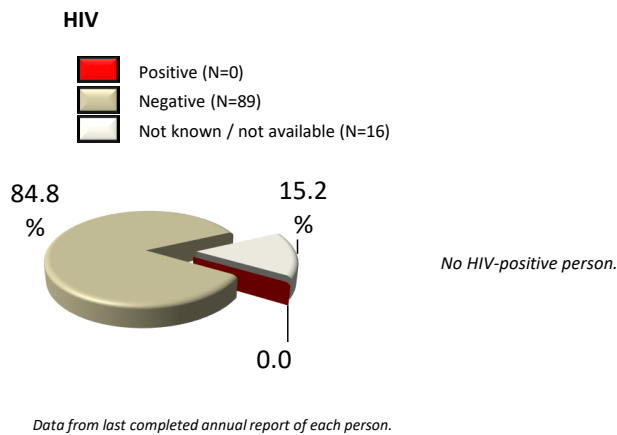
Hepatitis (ever) experienced

Adults
Haem
B
N=69



HIV

All
Haem B
N=105





Treatment outcomes and bleeding frequency Haemophilia B

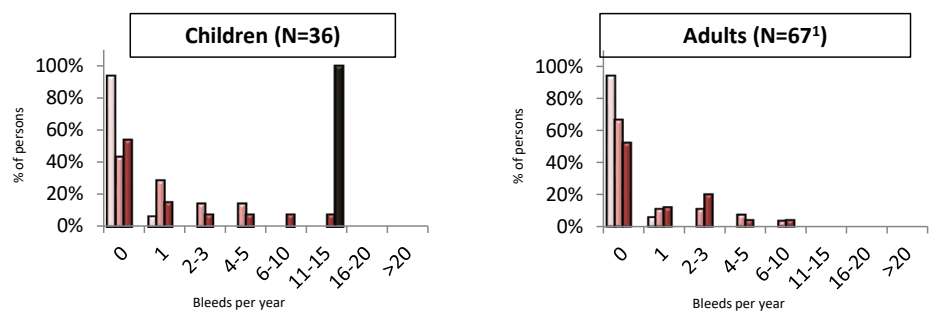
Data from year 2024 – sample size

All
Haem B
N=105

	Valid persons			Persons with valid annual report			Persons examined			Persons treated	
	N	%		N	%		N	%		N	%
All	105	100%	→	101	96.2%	→	81	77.1%	→	66	62.9%
of them with inhibitor	1			1			1			1	
Children	36	100%	→	36	100.0%	→	32	88.9%	→	22	61.1%
of them with inhibitor	1			1			1			1	
Adults	69	100%	→	65	94.2%	→	49	71.0%	→	44	63.8%
of them with inhibitor	0			0			0			0	

Frequency of bleeding requiring treatment in 2024

Haem B
N=103¹



Mild*	Moderate*	Severe*	Inhibitor	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
15	7	13	1	N valid	17	27	23	0
0.1	1.3	2.2	15.0	Mean	0.1	0.9	1.2	
0 (0 – 1)	1 (0 – 4)	0 (0 – 15)	15 (15 – 15)	Median (min – max)	0 (0 – 1)	0 (0 – 8)	0 (0 – 7)	
14 (93.3%)	3 (42.9%)	7 (53.8%)	0 (0%)	N (%) with no bleed	16 (94.1%)	18 (66.7%)	13 (52%)	

* without inhibitor

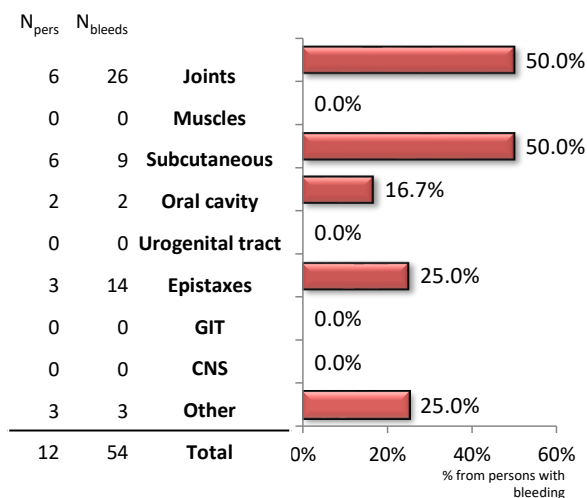
¹Frequency of bleeding is missing in 2 adults.

Location of bleeds in 2024

Children
n
Haem B
N=36

12 (33.3%) children experienced bleeding at least once in year; 54 bleeds were recorded in total, 4 bleeds required hospitalization. All of these 12 children have recorded location of their bleeds.

24 (66.7%) children recorded no bleed during year 2024.

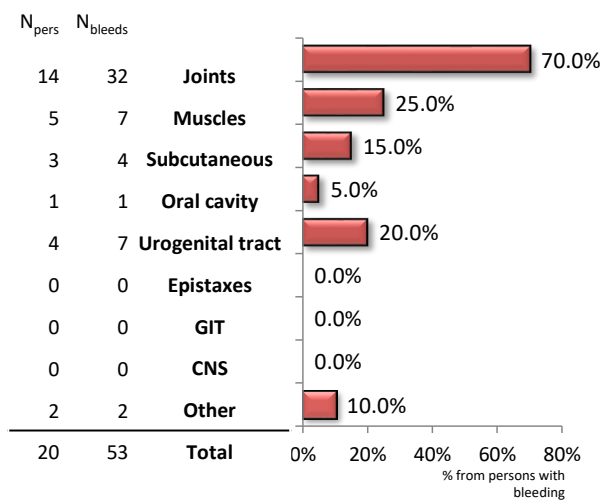


Location of bleeds in 2024

Adults
Haem
B
N=67¹

20 (29.9%) adults experienced bleeding requiring treatment at least once in year; 53 bleeds were recorded in total, 9 bleeds required hospitalization. All of these 20 adults have recorded location of their bleeds.

47 (70.1%) adults have recorded no bleed during year 2024.

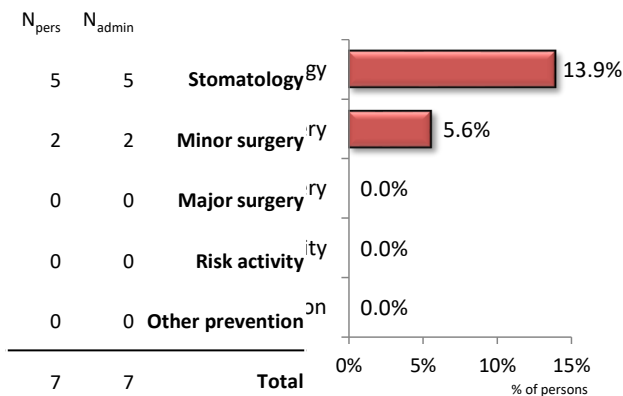


¹Frequency of bleeding is missing in 2 adults.

Preventive administration in 2024

Children
with
Haem B
N=36

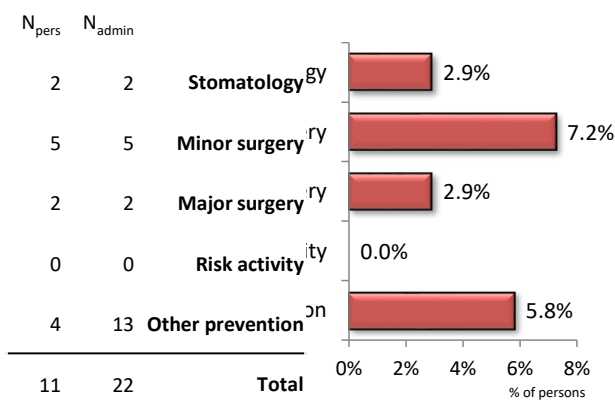
7 (19.4%) children were given factor to prevent bleeding during/before risk situation. 7 preventive administrations were recorded in total.



Preventive administration in 2024

Adults
Haem B
N=69

11 (15.9%) persons were given factor to prevent bleeding during/before risk situation. 22 preventive administrations were recorded in total.



This figure refers to preventive factor administration in adults with HB.



ABR according to treatment regimen Haemophilia B without inhibitor

Annual bleeding rate according to treatment regimen

Children
Haem B
N=35*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	15	0	6	1	3	10
Mean	0.1	0.0	1.5	0.0	1.7	2.4
Median (min – max)	0 (0 – 1)	(–)	1 (0 – 4)	0 (0 – 0)	1 (0 – 4)	0 (0 – 15)
Total no of recorded bleeds	1	0	9	0	5	24
Children on permanent prophylaxis	0 (0%)		1 (14.3%)		10 (76.9%)	
% of factor (FVIII) consumed by children on permanent prophylaxis	0.0%		64.8%		98.8%	

Location of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	15	0	6	1	3	10
JOINT BLEEDS						
Mean	0.0	0	0.8	0.0	1.0	0.7
Median (range)	0 (0 – 0)	(–)	0 (0 – 3)	0 (0 – 0)	0 (0 – 3)	0 (0 – 6)
Total no of recorded bleeds	0	0	5	0	3	7
OTHER BLEEDS						
Mean	0.1	0	0.7	0.0	0.7	1.7
Median (range)	0 (0 – 1)	(–)	1 (0 – 1)	0 (0 – 0)	1 (0 – 1)	0 (0 – 14)
Total no of recorded bleeds	1	0	4.00	0	2	17

* without inhibitor

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

The data on bleeding rate in children with HB. Median ABR in children with severe haemophilia B on prophylaxis decreased from 3 to 0 in 2024. Keep in mind low numbers of children with HB, though

Almost no joint bleeds in HB children in 2024.

Annual bleeding rate according to treatment regimen

Adults
Haem B
N=67*

Frequency of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	17	0	22	5	5	18
Mean	0.1	0.0	1.0	0.8	1.6	1.1
Median (min – max)	0 (0 – 1)	(–)	0 (0 – 8)	0 (0 – 4)	0 (0 – 5)	0 (0 – 7)
Total no of recorded bleeds	1	0	21	4	8	19
Adults on permanent prophylaxis	0 (0%)		5 (18.5%)		19 (76%)	
% of factor (FVIII) consumed by children on permanent prophylaxis	0.0%		51.5%		92.9%	

Location of bleeding	Mild*		Moderate*		Severe*	
Treatment regimen	OD	prophy	OD	prophy	OD	prophy
N valid	17	0	22	5	5	18
JOINT BLEEDS						
Mean	0.1	0	0.5	0.4	1.4	0.7
Median (range)	0 (0 – 1)	(–)	0 (0 – 6)	0 (0 – 2)	0 (0 – 4)	0 (0 – 4)
Total no of recorded bleeds	1	0	10	2	7	12
OTHER BLEEDS						
Mean	0.0	0	0.5	0.4	0.2	0.4
Median (range)	0 (0 – 0)	(–)	0 (0 – 4)	0 (0 – 2)	0 (0 – 1)	0 (0 – 3)
Total no of recorded bleeds	0	0	11	2	1	7

* without inhibitor; missing frequency of bleeding in 2 adults

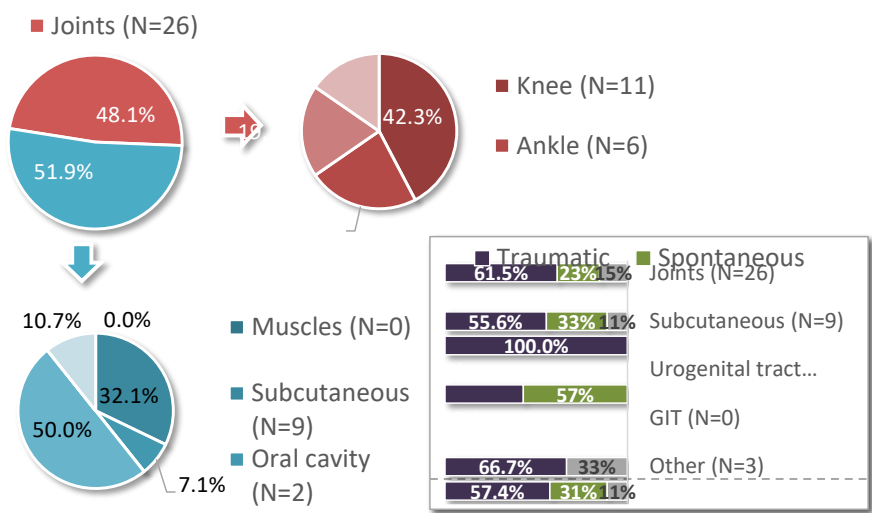
Treatment regimen:
OD = on demand and/or temporary prophylaxis
prophy = permanent prophylaxis

As well as in HA, adults with severe haemophilia B, who bleed frequently should be commenced on prophylaxis. Median ABR in adults with severe haemophilia B on prophylaxis decreased from 1 to 0 in 2024 for both ABR and JABR.

Location and etiology of bleeds

Children
Haem B
N=54*

* number of bleeds



Detailed treatment of bleeds

Children
Haem B
N=54*

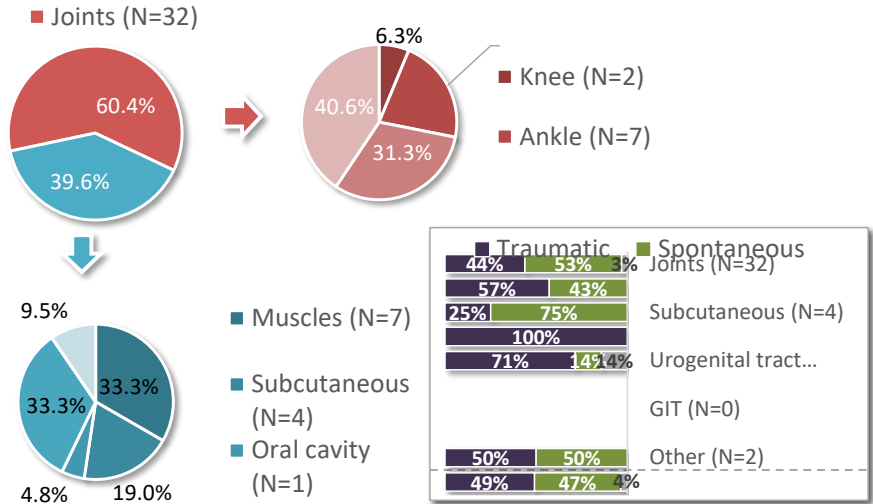
* number of bleeds

	Joints	Muscles	Subcuta- neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	26	0	9	2	0	14	0	0	3	54
FIX consumption per bleed (IU), valid N	13		5	2		7			3	30
geometric mean	2790.5		1974.4	1500.0		1059.6			1651.0	1913.1
median	3000.0		2000.0	1500.0		1000.0			1500.0	1750.0
min – max	1000–16000		1000–7500	1500–1500		500–2000			1000–3000	500–16000
sum	49 500		13 500	3 000		8 000			5 500	79 500
No. of doses per bleed										
geometric mean	2.1		1.5	1.7		1.2			1.3	1.7
median	2		1	2		1			1	1
min – max	0–8		0–5	1–3		0–3			1–2	0–8
Duration of therapy per bleed, days										
geometric mean	2.3		2.1	3.2		2.9			1.3	2.3
median	2		1	4		3			1	2
min – max	1–13		1–19	2–5		1–4			1–2	1–19
N (%) with hospitalization	2 (7.7%)		1 (11.1%)	1 (50%)		0 (0%)			0 (0%)	4 (7.4%)
N (%) with rebleeding	4 (15.4%)		3 (33.3%)	0 (0%)		2 (14.3%)			0 (0%)	9 (16.7%)

Location and etiology of bleeds

Adults
Haem B
N=53*

* number of bleeds



Detailed treatment of bleeds

Adults
Haem B
N=53*

* number of bleeds

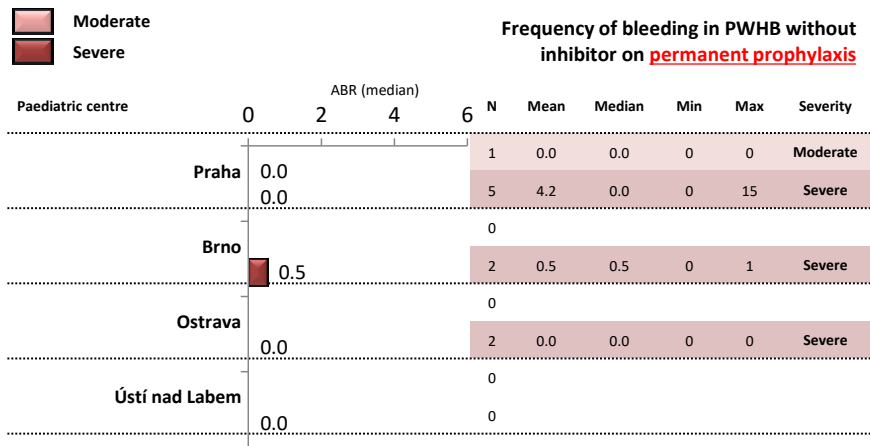
	Joints	Muscles	Subcuta- neous	Oral cavity	Urogenital tract	Epistaxes	GIT	CNS	Other	Total
No. of bleeds	32	7	4	1	7	0	0	0	2	53
FIX consumption per bleed (IU), valid N	31	7	4	1	7				2	52
geometric mean	5042.3	7416.1	4000.0	4000.0	10998.5				6324.6	5819.6
median	4000.0	8000.0	3000.0	4000.0	10000.0				7000.0	4000.0
min – max	2000–69000	2000–42000	2000–16000	4000–4000	2000–60000				4000–10000	2000–69000
sum	273 000	87 000	24 000	4 000	157 000				14 000	559 000
No. of doses per bleed										
geometric mean	3.2	3.1	1.2	2.0	3.0				1.0	2.8
median	1	2	1	2	2				1	1
min – max	0–2000	1–48	1–2	2–2	1–15				1–1	0–2000
Duration of therapy per bleed, days										
geometric mean	1.7	2.8	1.2	4.0	2.4				1.0	1.8
median	1	2	1	4	1				1	1
min – max	1–80	1–47	1–2	4–4	1–12				1–1	1–80
N (%) with hospitalization	3 (9.4%)	2 (28.6%)	0 (0%)	1 (100%)	3 (42.9%)				0 (0%)	9 (17%)
N (%) with rebleeding	1 (3.1%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)				0 (0%)	1 (1.9%)



ABR according to centres Haemophilia B (PWHB)

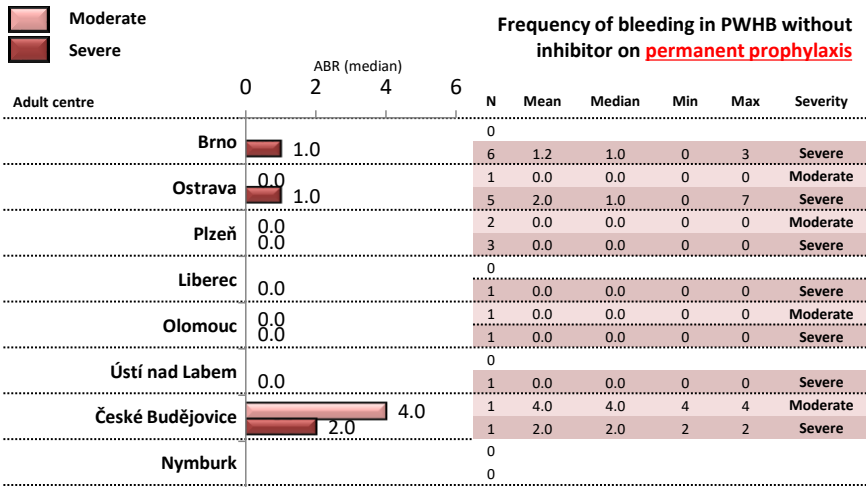
Annual bleeding rate on permanent prophylaxis

HaemB on prophylaxis
Paed. centres
N=10



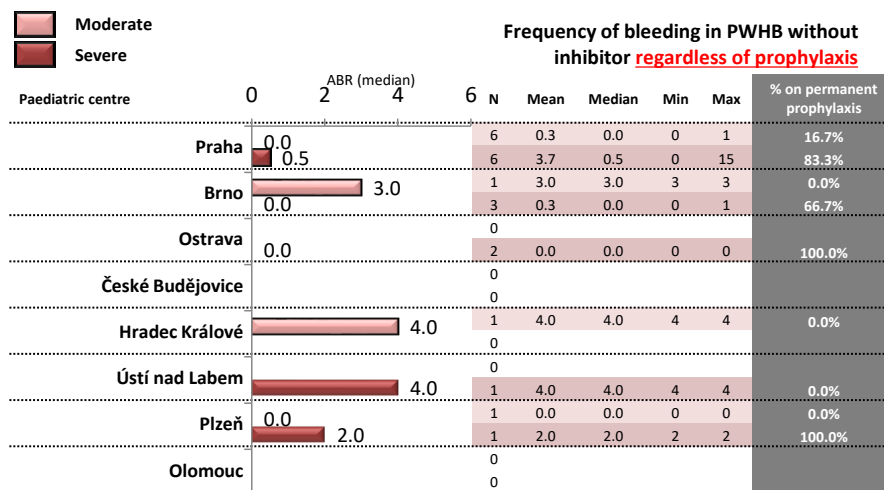
Annual bleeding rate on permanent prophylaxis

HaemB on prophylaxis
Adult centres
N=23



Annual bleeding rate regardless prophylaxis

HaemB
Paed. centres
N=22



Annual bleeding rate regardless prophylaxis

HaemB
Adult centres
N=48*

* missing ABR in 2 adults

 Moderate
 Severe

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

Adult centre	ABR (median)	N*	Mean	Median	Min	Max	% on permanent prophylaxis
Brno	1.0 2.0	5 7	0.6 1.7	1.0 2.0	0 0	1 5	0.0% 85.7%
Ostrava	0.0 1.0	3 7	1.3 1.4	0.0 1.0	0 0	4 7	33.3% 71.4%
Plzeň	0.0 0.0	3 3	0.0 0.0	0.0 0.0	0 0	0 0	66.7% 100.0%
Liberec	0.0 0.0	3 1	0.7 0.0	0.0 0.0	0 0	2 0	0.0% 100.0%
Olomouc	0.0 0.0	8 2	1.5 0.0	0.0 0.0	0 0	8 0	12.5% 33.3%
Ústí nad Labem	1.5	0 2	1.5	1.5	0	3	50.0%
České Budějovice	0.0 2.0	3 1	1.3 2.0	0.0 2.0	0 2	4 2	33.3% 100.0%
Nymburk		0 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	16.7%	1			17.9	17.9	0.0	0.0	5	0.4	0.0
	Severe	6	83.3%	5			45.2	55.6	4.2	0.0	1	1.0	1.0
Brno	Moderate	1	0.0%	0							1	3.0	3.0
	Severe	3	66.7%	2			44.3	44.3	0.5	0.5	1	0.0	0.0
Ostrava	Moderate	0	0.0%	0							0		
	Severe	2	100.0%	2			18.7	18.7	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	4.0	4.0
	Severe	0	0.0%	0							0		
Ústí nad Labem	Moderate	0	0.0%	0							0		
	Severe	1	0.0%	0							1	4.0	4.0
Plzeň	Moderate	1	0.0%	0							1	0.0	0.0
	Severe	1	100.0%	1			44.8	44.8	2.0	2.0	0		
Olomouc	Moderate	0	0.0%	0							0		
	Severe	0	0.0%	0							0		

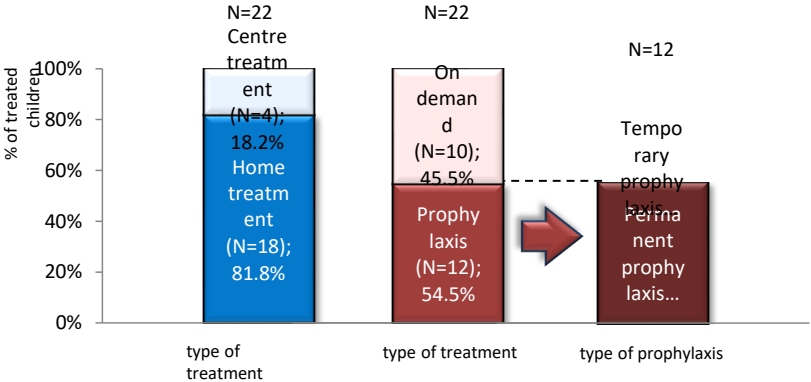
Prophylactic regimens and treatment outcomes

HaemB
Adult centres
N=50

Adult centre	Severity	Total N	% of patients	PERMANENT PROPHYLAXIS								ON-DEMAND / TEMPORARY PROPHY			
				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			Mean	Median	
Brno	Moderate	5	0.0%	0								5	0.6	1.0	52
	Severe	7	85.7%	6			37.5	34.2	1.2	1.0	35	1	5.0	5.0	22
Ostrava	Moderate	3	33.3%	1			29.6	29.6	0.0	0.0	25	2	2.0	2.0	63
	Severe	7	71.4%	5	50.6	50.6	47.6	45.2	2.0	1.0	57	2	0.0	0.0	54
Plzeň	Moderate	3	66.7%	2			23.5	23.5	0.0	0.0	58	1	0.0	0.0	62
	Severe	4	100.0%	4			29.3	29.6	0.0	0.0	45	0			
Liberec	Moderate	3	0.0%	0								3	0.7	0.0	42
	Severe	1	100.0%	1			76.9	76.9	0.0	0.0	32	0			
Olomouc	Moderate	8	12.5%	1			20.2	20.2	0.0	0.0	45	7	1.7	0.0	56
	Severe	3	33.3%	1			19.2	19.2	0.0	0.0	55	2	0.0	0.0	45
Ústí n. Labem	Moderate	0	0.0%	0								0			
	Severe	2	50.0%	1			19.2	19.2	0.0	0.0	29	1	3.0	3.0	53
Č. Budějovice	Moderate	3	33.3%	1			93.2	93.2	4.0	4.0	58	2	0.0	0.0	49
	Severe	1	100.0%	1			17.9	17.9	2.0	2.0	51	0			

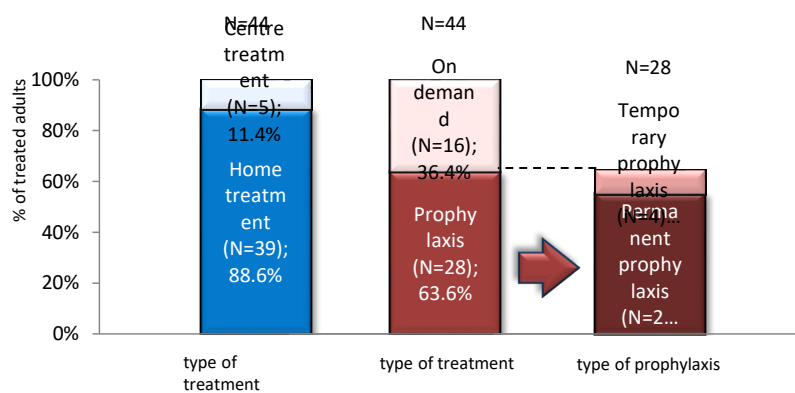
Type of treatment (subgroup of treated patients)

Children
on Haem B
N=22



Type of treatment (subgroup of treated patients)

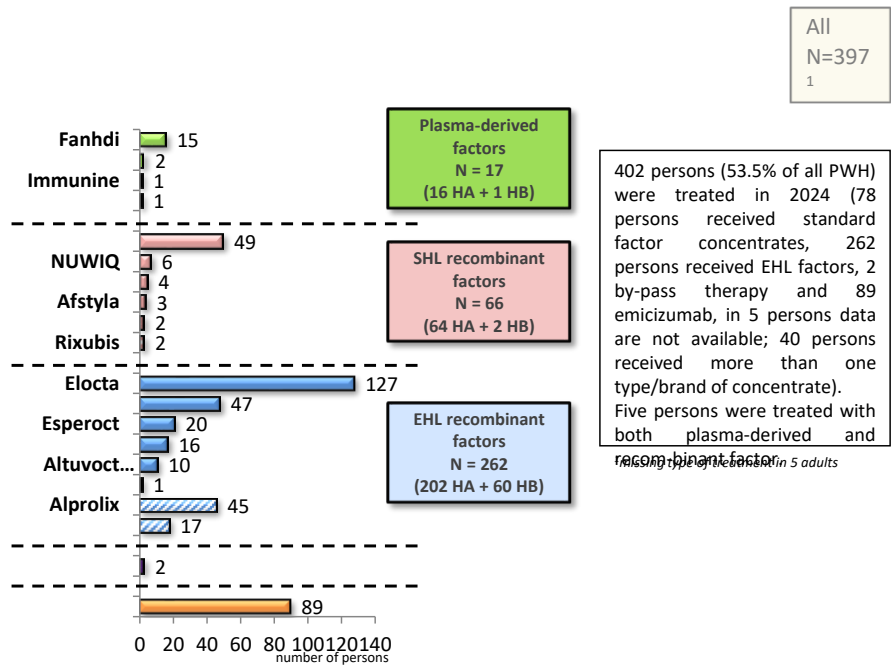
Adults
Haem
B
N=44





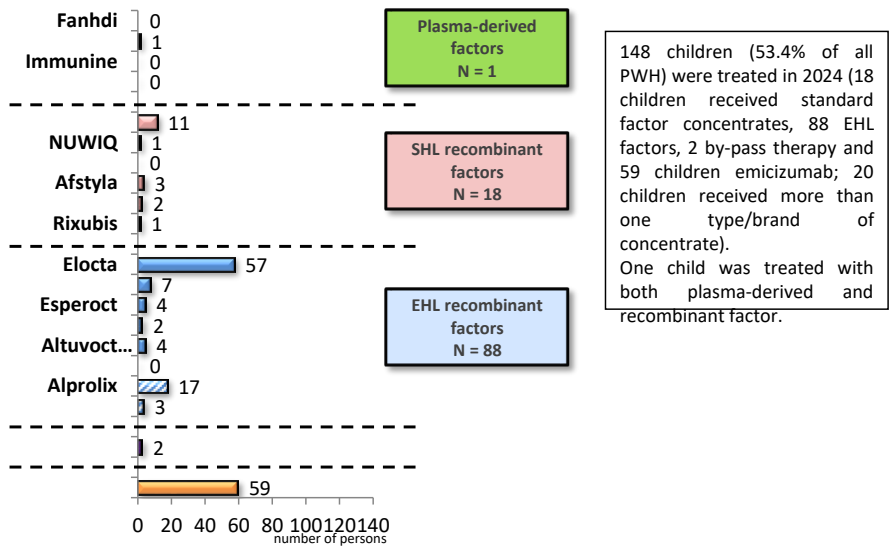
Treatment data and factor consumption Haemophilia A and B

Treatment



Treatment

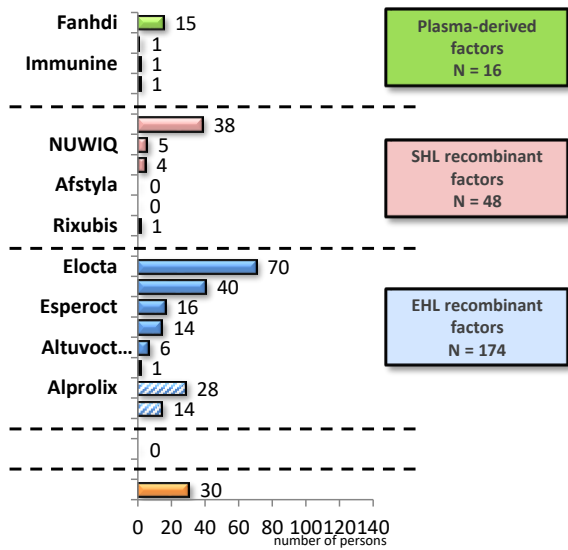
Children
n
N=148



Only 1 child registered in CNHP registry was treated with plasma-derived concentrates in 2024. Most children were switched to EHL, mainly Elocta. Majority of those treated with emicizumab (regardless of inhibitors) were children. Their number further increased (from 53 to 59)

Treatment

Adults
N=249
1



254 adults (53.5% of all PWH) were treated in 2024 (60 adults received standard factor concentrates, 174 EHL factors, and 30 adults received more than one type/brand of concentrate). Four adults were treated with both plasma-derived and recombinant factor.

¹missing type of treatment in 5 adults

Only 16 adults registered in CNHP registry were treated still with plasma derived concentrates in 2024 (was 23 in 2023). The number of adults treated with recombinants is further increasing over last years with most of them being treated with EHLs. Number of adults on emi increased from 23 to 30 in 2024

Comparison of treatment in years 2024 and 2023

All

	2024			2023		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All treated persons *	402	53.5	100.0	395	53.0	100.0
Plasma-derived factor	17	2.3	4.2	28	3.8	7.1
Recombinant factor	66	8.8	16.4	105	14.1	26.6
Recombinant f. EHL	262	34.8	65.2	238	31.9	60.3
Emicizumab	89	11.8	22.1	76	10.2	19.2
Without treatment	350	46.5	-	350	47.0	-
Total	752	100.0	-	745	100.0	-

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

Comparison of treatment in years 2024 and 2023

Children

	2024			2023		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All treated persons *	148	53.4	100.0	154	54.2	100.0
Plasma-derived factor	1	0.4	0.7	5	1.8	3.2
Recombinant factor	18	6.5	12.2	36	12.7	23.4
Recombinant f. EHL	88	31.8	59.5	83	29.2	53.9
Emicizumab	59	21.3	39.9	53	18.7	34.4
Without treatment	129	46.6	-	130	45.8	-
Total	277	100.0	-	284	100.0	-

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

Comparison of treatment in years 2024 and 2023

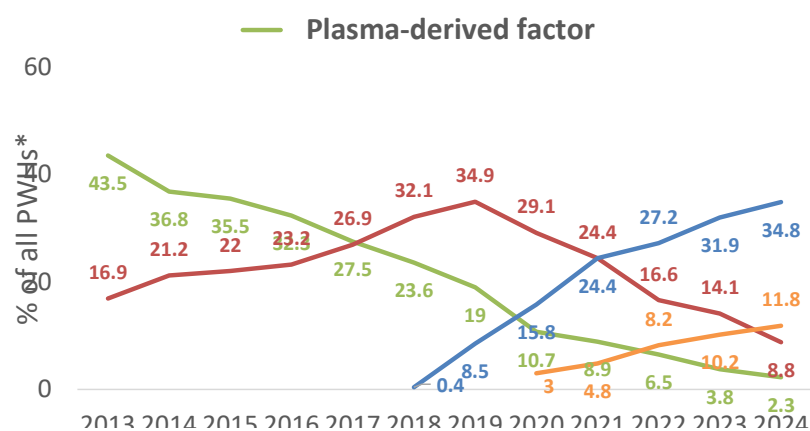
Adults

	2024			2023		
	N	% of all PWHs	% treated PWHs	N	% of all PWHs	% treated PWHs
All treated persons *	254	53.5	100.0	241	52.3	100.0
Plasma-derived factor	16	3.4	6.3	23	5.0	9.5
Recombinant factor	48	10.1	18.9	69	15.0	28.6
Recombinant f. EHL	174	36.6	68.5	155	33.6	64.3
Emicizumab	30	6.3	11.8	23	5.0	9.5
Without treatment	221	46.5	-	220	47.7	-
Total	475	100.0	-	461	100.0	-

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

Comparison of treatment in years

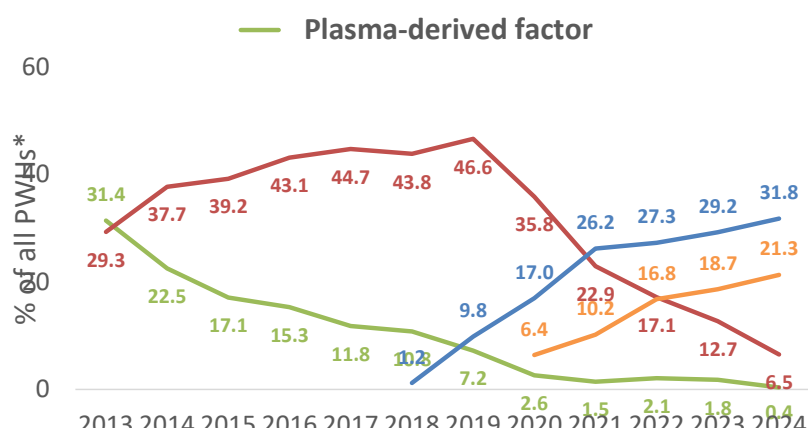
All



* One patient could have more type of treatments

Comparison of treatment in years

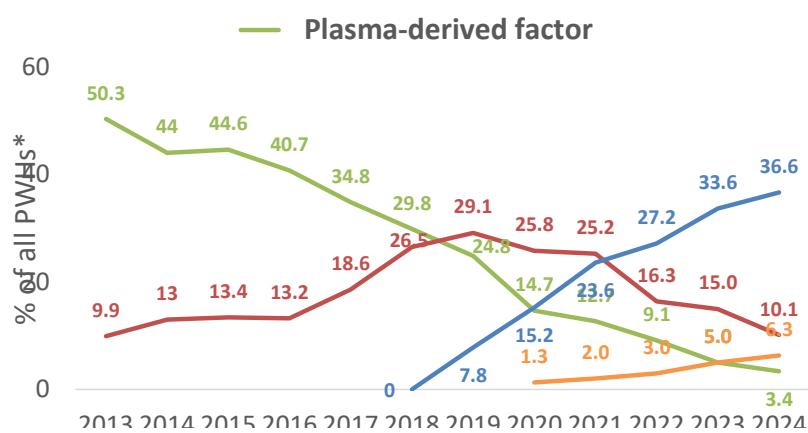
Children



* 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)	<i>Fanhdi</i>	1 379 150	14	98 510.7
	<i>Octanate</i>	215 000	2	107 500.0
	PD FVIII total	1 594 150	16	99 634.4
	<i>Advate</i>	1 940 267	49	39 597.3
	<i>NUWIQ</i>	1 282 950	6	213 825.0
	<i>NovoEight</i>	247 500	4	61 875.0
	<i>Afstyla</i>	607 500	3	202 500.0
	<i>Kovaltry</i>	5 500	2	2 750.0
	SHL REC FVIII total	4 083 717	64	63 808.1
	Standard FVIII total	5 677 867	79	71 872.7
	<i>Elocta</i>	21 014 066	124	169 468.3
	<i>Adynovi</i>	8 663 561	47	184 331.1
	<i>Esperoct</i>	3 252 800	20	162 640.0
	<i>Jivi</i>	3 158 995	16	197 437.2
	<i>Altuvect (Altuviiio)</i>	579 150	10	57 915.0
	<i>Other-recFVIII EHL</i>	213 900	1	213 900.0
	EHL REC FVIII total	36 882 472	202	182 586.5
	FVIII total	42 560 339	272	156 471.8
FIX (IU)	<i>Immunine</i>	0	0	
	<i>Octanine</i>	187 000	1	187 000.0
	FIX PD total	187 000	1	187 000.0
	<i>Rixubis</i>	112 000	2	56 000.0
	FIX REC total	112 000	2	56 000.0
	Standard FIX total	299 000	3	99 666.7
	<i>Alprolix</i>	4 719 447	45	104 876.6
	<i>Refixia</i>	1 032 640	15	68 842.7
	EHL REC FIX total	5 752 087	60	95 868.1
	FIX total	6 051 087	63	96 049.0
By-pass	<i>NovoSeven (mg)</i>	1 626	2	813.0
Emicizumab	<i>Hemlibra s.c. (mg)</i>	291 127	89	3 271.1

Consumption of drugs

Children

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	Fanhdii	0	0	
	Octanate	205 000	1	205 000.0
	PD FVIII total	205 000	1	205 000.0
	Advate	306 500	11	27 863.6
	NUWiq	28 000	1	28 000.0
	NovoEight	0	0	
	Afstyla	607 500	3	202 500.0
	Kovaltry	5 500	2	2 750.0
	SHL REC FVIII total	947 500	17	55 735.3
	Standard FVIII total	1 152 500	18	64 027.8
	Elocta	6 542 400	57	114 778.9
	Adynovi	1 140 500	7	162 928.6
	Esperoct	374 000	4	93 500.0
	Jivi	535 000	2	267 500.0
	Altuvocet (Altuviiio)	147 000	4	36 750.0
	Other-recFVIII EHL	0	0	
	EHL REC FVIII total	8 738 900	68	128 513.2
	FVIII total	9 891 400	85	116 369.4
FIX (IU)	Immunine	0	0	
	Octanine	0	0	
	FIX PD total	0	0	
	Rixubis	8 000	1	8 000.0
	FIX REC total	8 000	1	8 000.0
	Standard FIX total	8 000	1	8 000.0
	Alprolix	652 000	17	38 352.9
	Refixia	169 000	3	56 333.3
	EHL REC FIX total	821 000	20	41 050.0
	FIX total	829 000	21	39 476.2
By-pass	NovoSeven (mg)	1 626.0	2	813.0
Emicizumab	Hemlibra s.c. (mg)	102 306	59	1 734.0

Consumption of drugs

Adults

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person
FVIII (IU)	<i>Fanhdii</i>	1 379 150	14	98 510.7
	<i>Octanate</i>	10 000	1	10 000.0
	PD FVIII total	1 389 150	15	92 610.0
	<i>Advate</i>	1 633 767	38	42 983.9
	<i>NUWiq</i>	1 254 950	5	250 990.0
	<i>NovoEight</i>	247 500	4	61 875.0
	<i>Afstyla</i>	0	0	
	<i>Kovaltry</i>	0	0	
	SHL REC FVIII total	3 136 217	47	66 728.0
	Standard FVIII total	4 525 367	61	74 186.3
	<i>Elocta</i>	14 471 666	67	215 995.0
	<i>Adynovi</i>	7 523 061	40	188 076.5
	<i>Esperoct</i>	2 878 800	16	179 925.0
	<i>Jivi</i>	2 623 995	14	187 428.2
	<i>Altuvect (Altuviiio)</i>	432 150	6	72 025.0
	<i>Other-recFVIII EHL</i>	213 900	1	213 900.0
	EHL REC FVIII total	28 143 572	134	210 026.7
	FVIII total	32 668 939	187	174 700.2
FIX (IU)	<i>Immunine</i>	0	0	
	<i>Octanine</i>	187 000	1	187 000.0
	FIX PD total	187 000	1	187 000.0
	<i>Rixubis</i>	104 000	1	104 000.0
	FIX REC total	104 000	1	104 000.0
	Standard FIX total	291 000	2	145 500.0
	<i>Alprolix</i>	4 067 447	28	145 266.0
	<i>Refixia</i>	863 640	12	71 970.0
	EHL REC FIX total	4 931 087	40	123 277.2
	FIX total	5 222 087	42	124 335.4
By-pass	<i>NovoSeven (mg)</i>	0.0	0	
<i>Emicizumab</i>	<i>Hemlibra s.c. (mg)</i>	188 821	30	6 294.0

Absolute numbers of respective concentrates in this figure refer ONLY to the records within CNHP registry, which have been updated in 2023. The most important information on this slide is „Average annual consumption per treated person“. This reflects nation-wide consumption of factor concentrate per treated PWH.

Though in rFVIII SHLs the dosing is almost identical for children and adults, in rFVIII EHLs it is significantly different, reflecting the difference in body weight between adults and children. Thus it seems, that EHLs are doses well in both adults and children, but adults with SHLs are still underdosed.