Hemofilie v ČR
Výsledky a úhrada léčby

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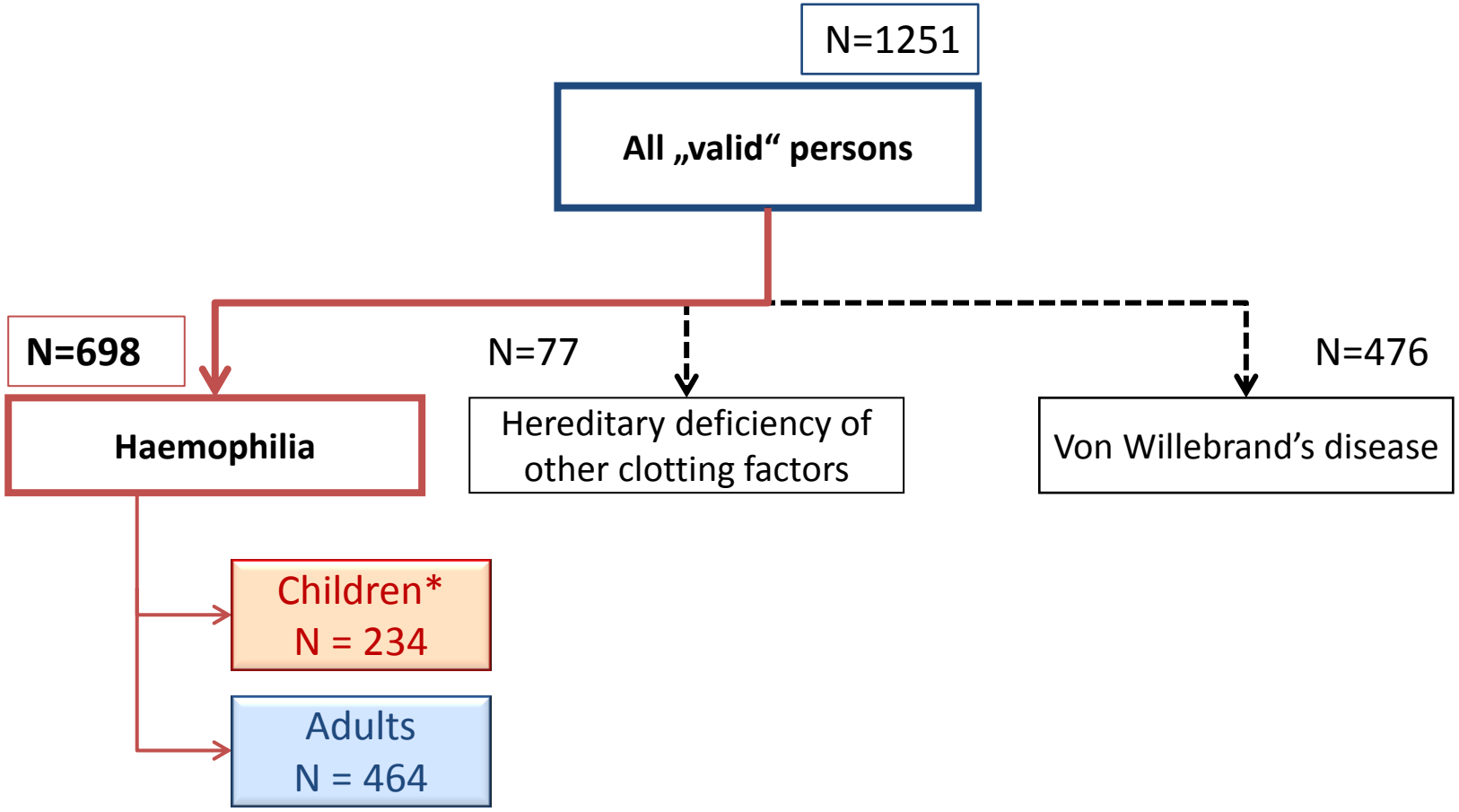
jménem a pro
Centra sdružená v Českém národním hemofilickém programu

Demografie osob s hemofilií v ČR (ČNHP data)

Sample size - Haemophilia

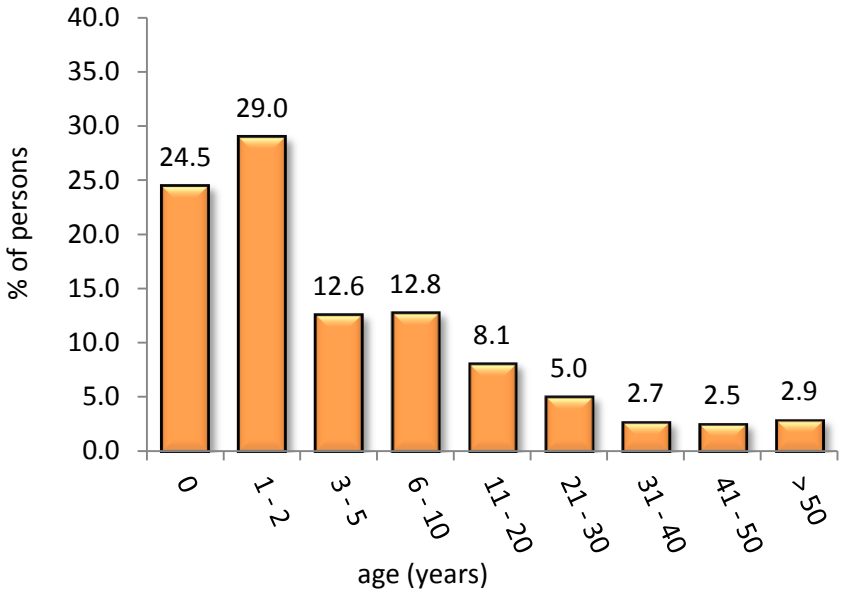


* *Persons under 19 years old in 2014*

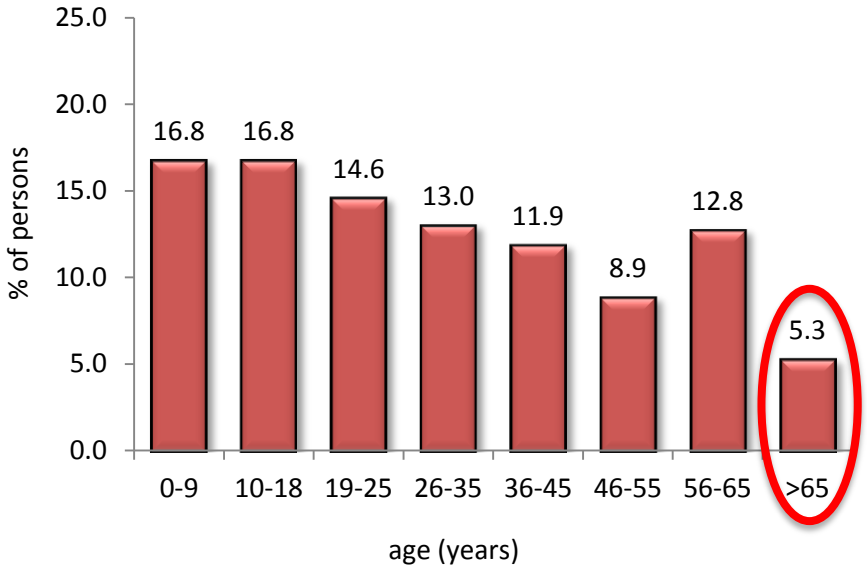
Age

All
N=698

	Age at diagnosis (years)
N	*556**
Mean	*8.1*
Median (min - max)	*2 (0 – 81)*



	Current age (years)
N	*698*
Mean	*31.2*
Median (min - max)	*27 (0 – 92)*

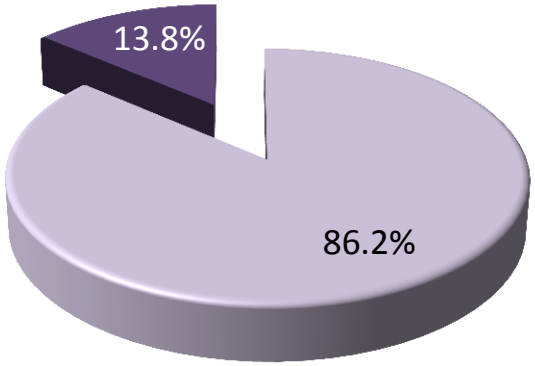


** Missing information on year of diagnosis in 142 persons.*

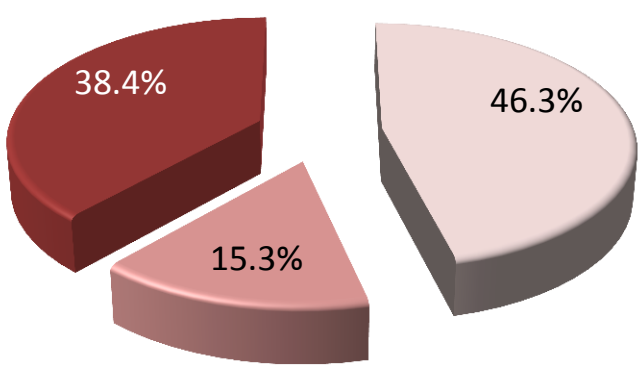
Type and severity of haemophilia I

All
N=698

Type of haemophilia



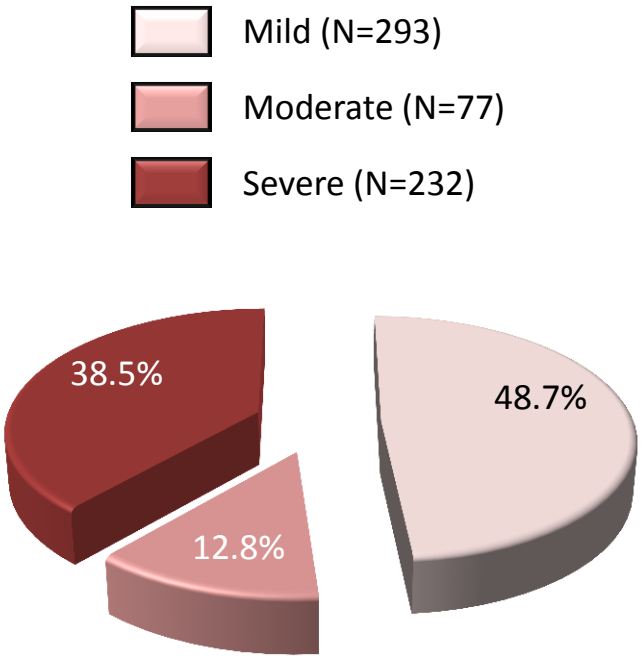
Severity of haemophilia



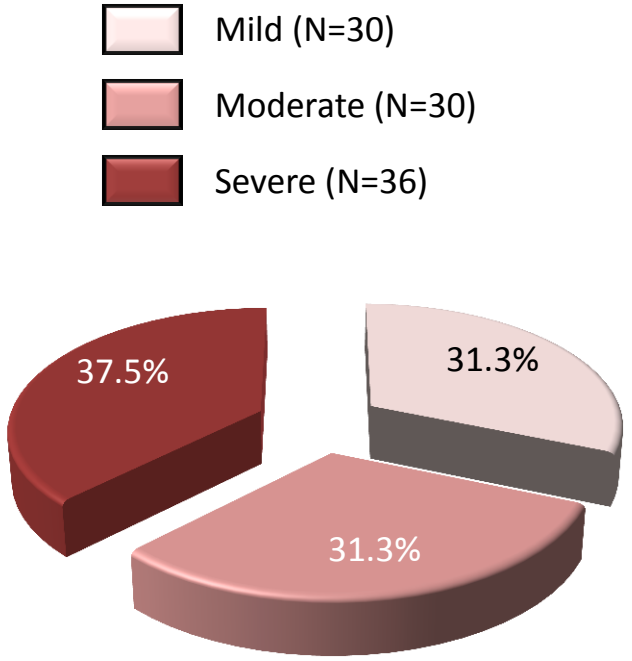
Type and severity of haemophilia II

All
N=698

Haemophilia A (N=602)



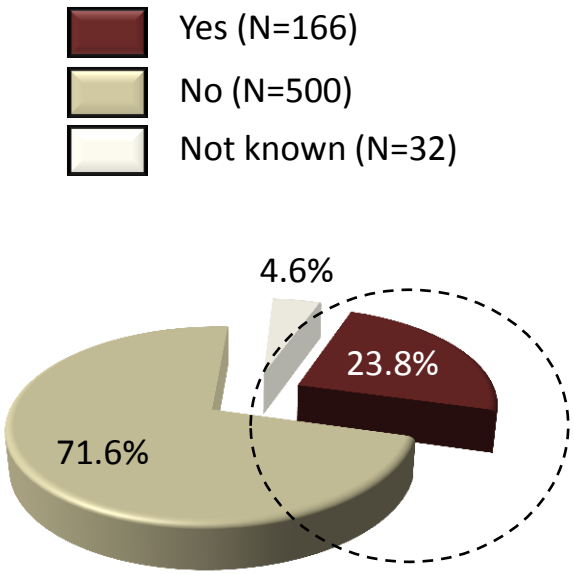
Haemophilia B (N=96)



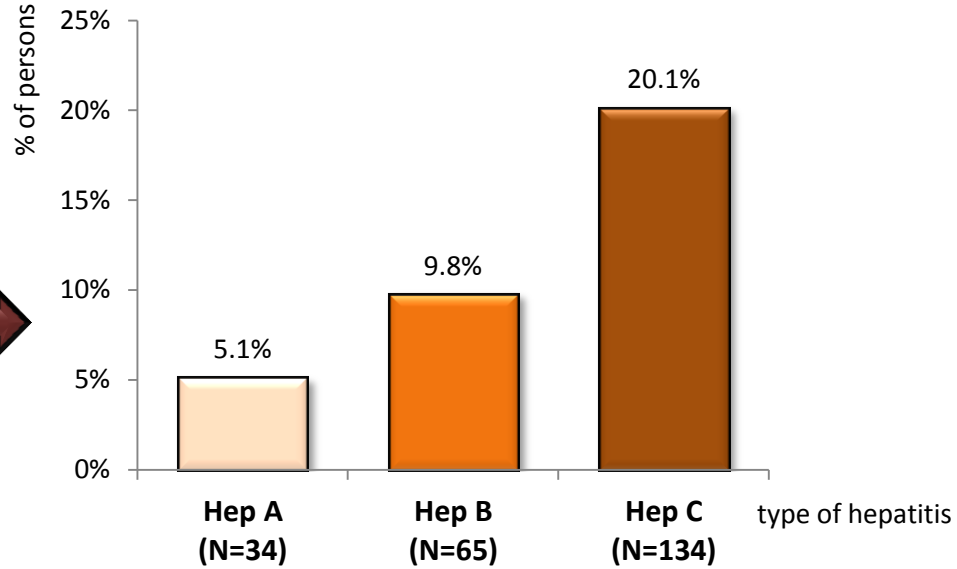
Hepatitis experienced

All
N=698

Experienced hepatitis



N=166*



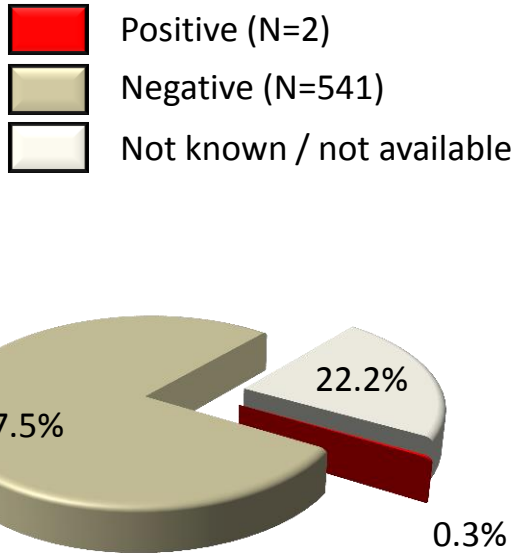
Data from last annual report of each person.

**Total of 233 cases of hepatitis in 166 persons. One person may have more types of hepatitis recorded.*

HIV

All
N=698

HIV



N=2 (+ 1 in another centre)

All HIV-positive persons are adults.

Data from last annual report of each person.

All
N=698

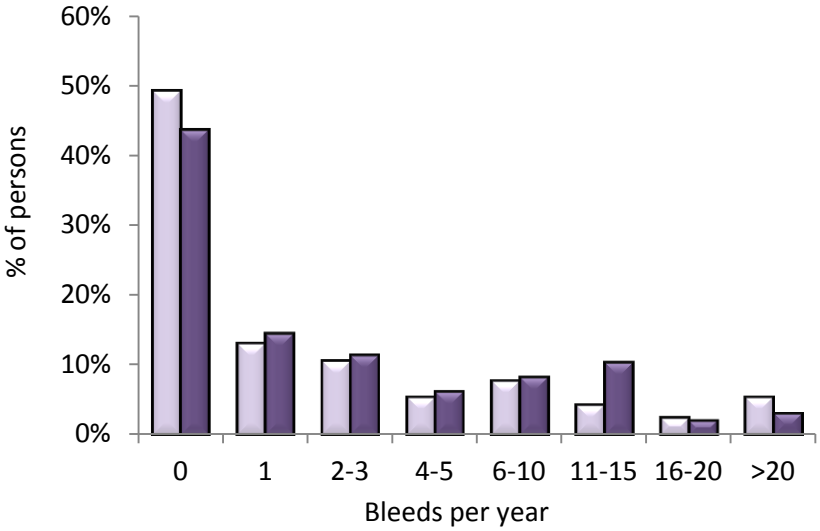
Persons with haemophilia with inhibitors in year 2014

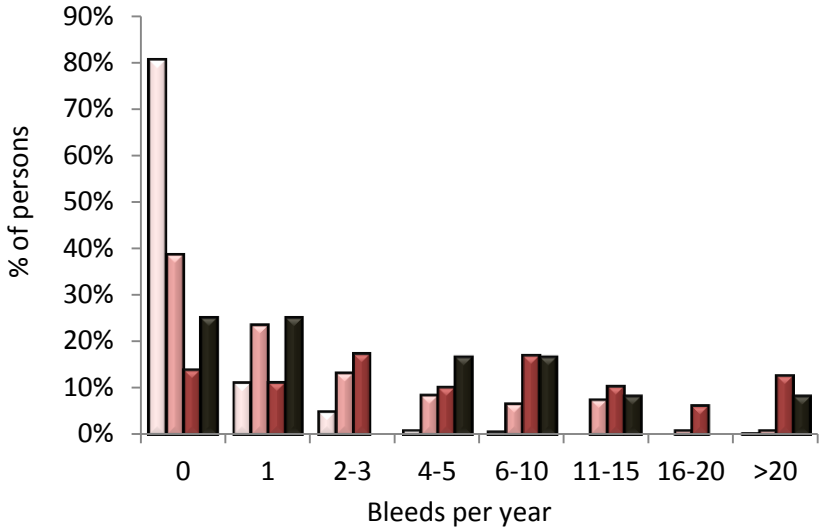
- Active inhibitors were recorded in **12 persons** in year 2014 (+ 6 adults in another centre)
  - 6 children and 6 adults
  - 11 haemophilia A and 1 heamophilia B
  - 10 HR and 2 LR
  - 4 patients were treated with rFVIIa, 2 patients with aPCC, other 2 patients with both rFVIIa and aPCC
  - 2 patients were without „by-pass“ therapy and 2 patients were without any recorded treatment

Léčba a její výsledky (ČNHP data)

Frequency of bleeding requiring treatment in 2014

All
N=692





Haemophilia A	Haemophilia B	Frequency of bleeding	Mild*	Moderate*	Severe*	Inhibitor
595[1]	*96*	**N valid**	*317*	*106*	*256[1]*	*12*
4.1	*4.7*	**Mean**	***0.4***	***2.8***	***9.4***	***5.5***
0.5 (0 – 55)	*1 (0 – 72)*	**Median (min – max)**	*0 (0 – 24)*	*1 (0 – 21)*	*5 (0 – 72)*	*2.5 (0 – 27)*

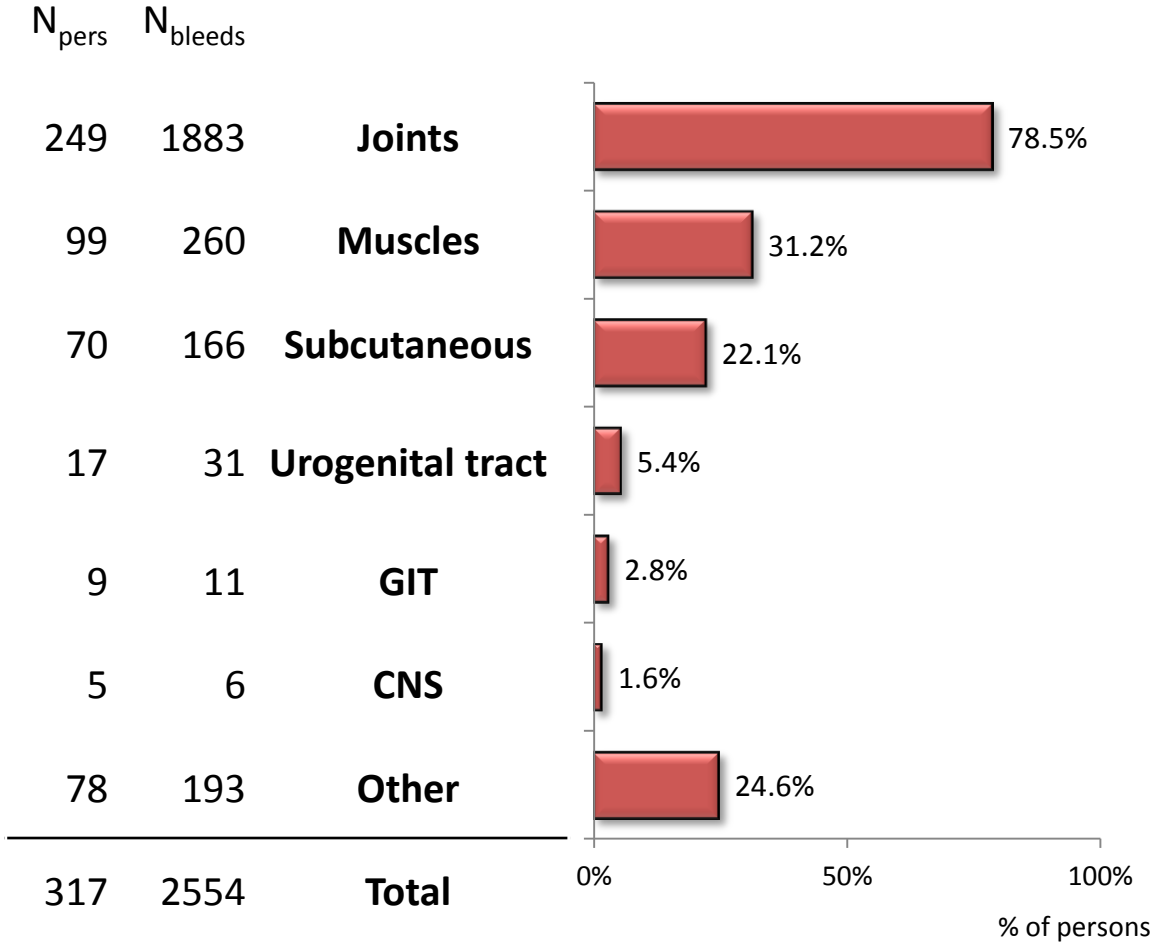
[1]Frequency of bleeding is missing in 1 adult.

** without inhibitor*

Location of bleeds in 2014

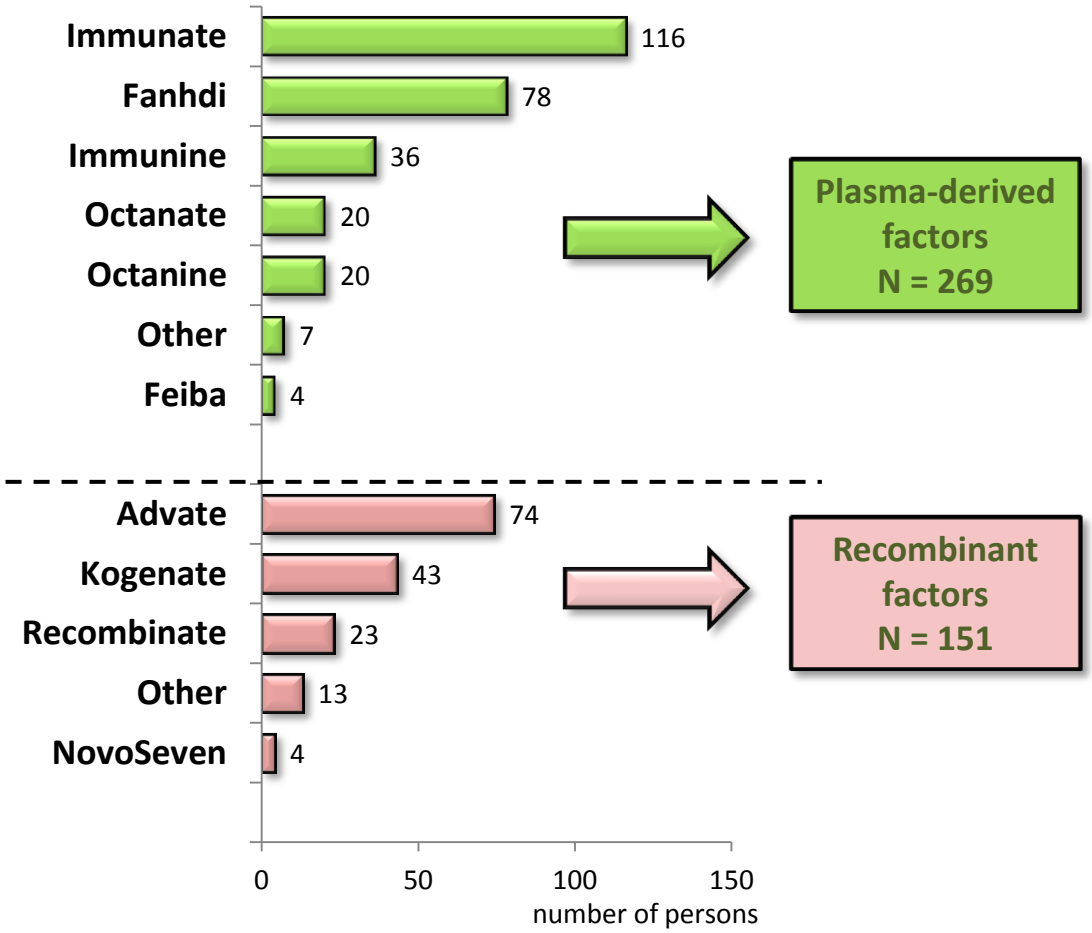
All
N=692

353 (51%) persons experienced bleeding requiring treatment at least once per year; 2889 bleeds were recorded in total, 86 bleeds required hospitalization.
317 of these 353 persons have recorded location of their bleeds. Localization is not known in 36 persons.
339 (49%) persons recorded no bleed during year 2014.



All
N=402

Treatment

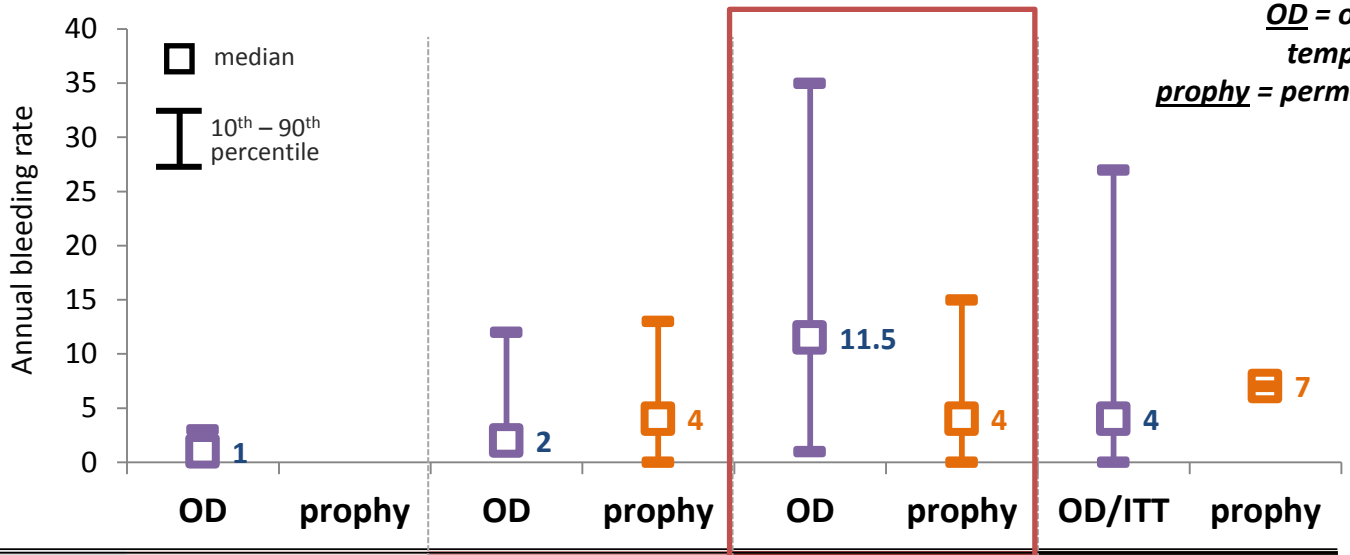


402 persons (58.3% of all PWHs) received factor concentrates in 2014 (34 of them received more than one type/make of concentrate).
Plasma-derived factors were administered more frequently – in 269 persons (39% of all PWHs, 66.9% of treated PHWs), whereas recombinant factors in 151 persons (21.9% of all PWHs, 37.6% of treated PHWs).
18 persons were treated with both plasma-derived and recombinant factor.

Annual bleeding rate according to treatment regimen

All
N=402

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



Frequency of bleeding	Mild*		Moderate*		Severe*		Inhibitor	
N total	79	0	51	19	98[1]	145	9	1
Mean	1.6		4.1	4.6	15.1	6.6	6.6	7
Median (min – max)	1 (0 – 24)		2 (0 – 21)	4 (0 – 17)	11.5 (0 – 55)	4 (0 – 72)	4 (0 – 27)	7
Total no of recorded bleeds	123		211	88	1451	950	59	7
persons on permanent prophylaxis	0		19 (27.1%)		145 (59.7%)		1 (10%)	
% of drugs (FVIII and FIX) consumed by persons on permanent prophylaxix	-		66.2 %		76.5 %		26.5 %	

* without inhibitor

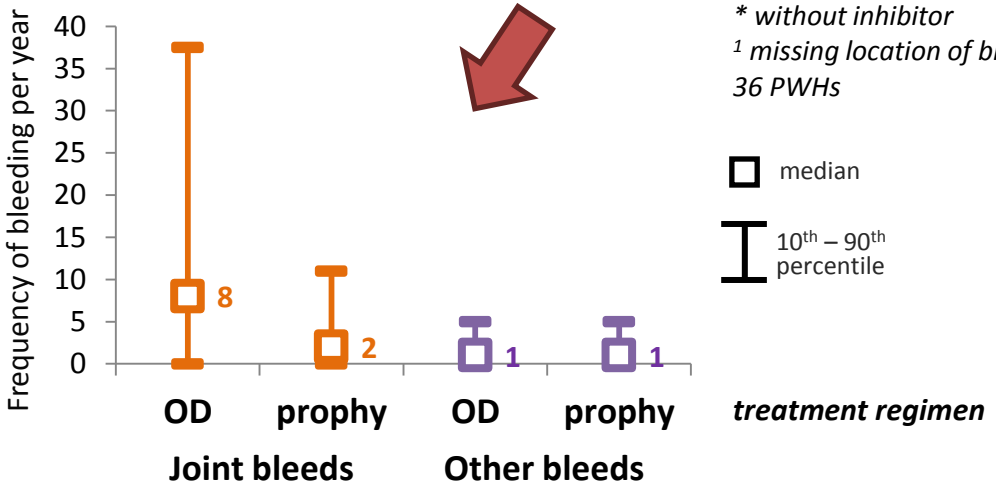
[1] Frequency of bleeding is missing in 1 adult.

Czech National Hemophilia Program

Joint and other bleeds according to treatment regimen

All
N=366[1]

Frequency of bleeding	Mild*		Moderate*		Severe*		Inhibitor	
Treatment regimen	*OD*	*prophy*	*OD*	*prophy*	*OD*	*prophy*	*OD/ITT*	*prophy*
N valid	*72*	*0*	*48*	*19*	*80*	*138*	*8*	*1*
JOINT BLEEDS								
Mean	*0.6*		*2.2*	*3.2*	***12.6***	***4.6***	*3.6*	*2.0*
Median (range)	*0 (0 – 5)*		*0 (0 – 14)*	*2 (0 – 13)*	***8 (0 – 55)***	***2 (0 – 64)***	*1 (0 – 16)*	*2*
Total no of recorded bleeds	*41*		*103*	*61*	***1009***	***638***	*29*	*2*
OTHER BLEEDS								
Mean	*0.9*		*2.1*	*1.4*	***2.0***	***2.1***	*3.3*	*5.0*
Median (range)	*0 (0 – 22)*		*0 (0 – 17)*	*1 (0 – 7)*	***1 (0 – 19)***	***1 (0 – 31)***	*2 (0 – 12)*	*5*
Total no of recorded bleeds	*66*		*98*	*26*	***159***	***287***	*26*	*5*



** without inhibitor*
[1] missing location of bleeds in 36 PWHs

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

All

Consumption of drugs

	Drug (IU)	Total annual consumption	Number of treated persons	Average annual consumption per treated person	Number of valid persons	Average annual consumption per valid person
FVIII	Immunate	8 858 600	116	76 367.2	602	14 715.3
	Fanhdi	6 622 350	78	84 901.9		11 000.6
	Octanate	2 304 000	20	115 200.0		3 827.2
	Other plasma-derived	2 130 850	7	304 407.1		3 539.6
	Advate	6 517 104	74	88 069.0		10 825.8
	Kogenate	3 950 750	43	91 877.9		6 562.7
	Recombinate	1 817 500	23	79 021.7		3 019.1
	Other recombinant	1 885 050	9	209 450.0		3 131.3
	FVIII total (IU)	**34 086 204**	**340**	**100 253.5**		**56 621.6**
FIX	Immunine	1 892 700	36	52 575.0	96	19 715.6
	Octanine	2 922 000	20	146 100.0		30 437.5
	Other recombinant	600 820	4	150 205.0		6 258.5
	FIX total (IU)	**5 415 520**	**58**	**93 371.0**		**56 411.7**
aPCC	Feiba	252 000	4	63 000.0		
rFVIIa	NovoSeven (mg)	1 715 mg	6	285.8 mg		
Plasma-derived factors - TOTAL*		**24 730 500**	**266**	**92 971.8**	698	**35 430.5**
Recombinant factors - TOTAL*		**14 771 224**	**145**	**101 870.5**		**21 162.2**
TOTAL CONSUMPTION (IU)*		**39 501 724**	**398**	**99 250.6**		**56 592.7**

- plasma-derived factors = Immunate, Fanhdi, Octanate, Immunine, Octanine, Other plasma-derived
- recombinant factors = Advate, Kogenate, Recombinate, BAX 326, Other recombinant

*TOTAL CONSUMPTION = all mentioned drugs excluding Feiba and NovoSeven

Kolik nás to stojí?

Celkové počty osob s hemofilií v ČR
Data WFH 2014

(Please DO NOT estimate or guess)	Number	Not known
1. Total number of identified people with **hemophilia A or B,** or **type unknown** (PWH)	1066	☐
2. Number of identified people with **von Willebrand disease** (VWD)	804	☐
3. Number of identified people with other hereditary bleeding disorders (including rare factor deficiencies and inherited platelet disorders. See question 6 for the list of specific disorders.)	77	☐
Do you consider these numbers to be accurate?	Yes ☒	Not sure ☐

Age group	Number with hemophilia A	Number with hemophilia B	Number with hemophilia type unknown	Number with VWD
0 - 4 years old	41	7	0	10
5 - 13 years old	102	19	0	65
14 - 18 years old	57	8	0	40
19 - 44 years old	432	51	0	371
45 years or older	298	51	0	318
Patients with age Unknown	0	0	0	0
No age data	☐	☐	☐	☐

Kvalifikovaný odhad nákladů na léčbu hemofilie A v ČR v roce 2014

- 930 osob s hemofilií A
  - Z nich cca 40% (370) potřebuje nákladnou léčbu
- Spotřebujeme cca 50 mil IU FVIII ročně
  - Cena 1 IU cca 9 CZK
- Nakoupíme FVIII cca za 450 mil CZK
  - Náklady na lék tvoří >90% nákladů na léčbu
- Náklady na léčbu hemofilie **A bez komplikací** se pohybují kolem 500-550 mil CZK/rok
- Osob s hemofilií B je 136 (40% s nákladnou léčbou), jejich léčba je levnější a s méně komplikacemi
  - Náklady na léčbu hemofilie B se mohou pohybovat do 15% nákladů na Hemofilii A
- Náklady na léčbu komplikací (zejména inhibitoru)
  - Mohou být od desítek po stovky mil CZK
- ***Celkem se náklady na léčbu hemofilie v ČR pohybují ročně odhadem kolem ¾ mld CZK (pro cca 400-450 osob)***

Paradigma

- Léčba hemofilie je:
  - Extrémně nákladná, je-li vztažena na jednotlivce (***"rare disease"***)
  - Celkově však není suma příliš vysoká v celonárodním měřítku
- Úhrada léčby hemofilie se vždy bude ***vymykat*** jakémukoli paušálu (vč ***DRG***)
- Léčba hemofilie musí být ***plně hrazena*** (jinak systém zkolabuje), nicméně úhrada má smysl jen v ***rámci definované sítě center***
- ***Počet*** PWHs je a bude ***stabilní***
- ***Léky*** proti hemofilii ***nelze podat jiné dg***
  - ***Benevolentní systém úhrady NELZE zneužít***
- ***Léčba*** hemofilie je tedy dobře ***definovatelná, predikovatelná a kontrolovatelná***!

Doporučení
(z pohledu hematologa)

- Léčba hemofilie by měla i nadále probíhat (a být hrazena) v síti center (audity MZd, ZP, ČHS)
- Antihemofilika by i nadále měla být vyčleněna ve zvláštní příloze UV (t.č. příloha 14)
- Ponechme v budoucích ÚV tuto větu:
  - *Léčivé přípravky vyjmuté z platby případovým paušálem a uvedené v příloze č. 14 k této vyhlášce uhradí zdravotní pojišťovna poskytovateli výkonově ve výši jejich vykázané jednotkové ceny, maximálně však ve výši jejich vykázané jednotkové ceny v referenčním období.*

- *Podpořeno z dotačního programu MZ: Národní akční plány a koncepce*