WFH
WORLD FEDERATION OF HEMOPHILIA
FÉDÉRATION MONDIALE DE L'HÉMOPHILIE
FEDERACIÓN MUNDIAL DE HEMOFILIA

Annual Global Survey 2017

Please return to WFH by May 30, 2018

A. National Hemophilia Organization

Organization name	Czech Society of Hemophiliacs
City	Prague
Country	Czech Republic
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This form completed by:	First name Martin Last name Bohůn Email m.bohun@hemofilici.cz

Please Click Here **to validate Organization contact information**

The WFH would like to know how you collect the data you are providing for this survey. If you have a registry, we would like to know more about the registry. A registry is a regularly updated centralized list of identified people with hemophilia (PWH) or inherited bleeding disorders. A registry includes information on personal details, diagnosis, treatment, and complications**.**

What is the source of the numbers provided for this survey?	**Check one** ☐ Hemophilia Society and/or NMO registry or database ☒ Hospital(s)/HTC(s) registry or database ☐ Health Ministry registry or database ☐ Other (please describe):
How often is your database updated?	☒ Ongoing update (can be updated anytime) ☐ Yearly update (the registry is updated once each year) ☐ Other (please describe):
Who updates the database?	☒ Doctors update the database ☐ Patient organization updates the database ☐ Hospitals or clinics update the database ☐ Other (please describe):
Have all the identified patients in your country been included in this report? If not, please explain.	Yes ☒ No ☐ Please explain:

Please Click Here **to validate Data source**

B. Identified Patients

(Please DO NOT estimate or guess)	**Number**	**Not known**
1. Total number of identified people with **hemophilia A or B,** or **type unknown** (PWH)	1077	☐
2. Number of identified people with **von Willebrand disease** (VWD)	837	☐
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.)	132	☐
Do you consider these numbers to be accurate?	Yes ☒	Not sure ☐

Please Click Here **to validate number of patients**

4. Number of people with Hemophilia and von Willebrand disease by age group

Age group	Number with hemophilia A	Number with hemophilia B	Number with hemophilia type unknown	Number with VWD
0 - 4 years old	39	9	0	9
5 - 13 years old	110	16	0	59
14 - 18 years old	55	8	0	49
19 - 44 years old	420	50	0	379
45 years or older	312	58	0	341
Patients with age Unknown				
No age data	☐	☐	☐	☐

The age distribution of Hemophilia A, B and unknown should be equal to the number of PWH in question B1
The age distribution of vWD should be equal to the number of vWD in question B2

Do you consider these numbers to be accurate?	Yes ☒	Not sure ☐
5. Do you collect age data in a format that does not match question 4? (If you do collect age data in another format, please send it to the WFH in a separate attachment.)		Yes ☐

Please Click Here **to validate Age section**

6. Type of hereditary bleeding disorder

The sum of *Male, Female,* and *Gender Unknown* should be equal to Total.

Diagnosis	Total	Male	Female	Gender unknown	No data
Hemophilia A	936	936	0	0	☐
Hemophilia B	141	141	0	0	☐
Hemophilia, type unknown	0	0	0	0	☐
von Willebrand disease	837	253	331	253	☐
Factor I deficiency	0	0	0	0	☐
Factor II deficiency	2	0	2	0	☐
Factor V deficiency	7	1	6	0	☐
Factor V+VIII deficiency	0	0	0	0	☐
Factor VII deficiency	65	34	31	0	☐
Factor X deficiency	4	3	1	0	☐
Factor XI deficiency	22	10	12	0	☐
Factor XIII deficiency	1	0	1	0	☐
Rare factor deficiency: type unknown	31	9	22	0	☐
Platelet disorders: Glanzmann thrombasthenia					☒
Platelet disorders: Bernard Soulier Syndrome					☒
Platelet disorders: other or unknown					☒

The sum of Totals Hemophilia A, B, and type unknown should be equal to the number of PWH in question B1.
The Total of vWD should be equal to the number of vWD in question B2.
The sum of Total of the all other bleeding and platelets disorders should be equal to the number of OBD in question B3
A woman who has ≤40%percent of the normal level of clotting factor would be considered a person with hemophilia. A woman with more than 40% FVIII is considered a carrier and should not be included in this report.

Do you consider these numbers to be accurate?	Yes ☒	Not sure ☐

Please Click Here **to validate Gender section**

7. How are patients with rare bleeding disorders (deficiency in FI, FII, FV, FV+VIII, FVII, FX, FXI FXIII) classified?

Factor level measurements ☒	Clinical diagnosis ☐ (bleeding, family history)	Other ☐ (please describe):	No data ☐

How are patients with von Willebrand Disease classified?

Factor level measurements ☒	Severe bleeding symptoms ☐	Other ☐ (please describe):	No data ☐

8. Number of identified people with hemophilia by gender and severity

There are three levels of **severity** of hemophilia: **mild, moderate,** and **severe**. The severity of hemophilia depends on the amount of clotting factor in the person's blood.

- A person (male or female) with >5-40 per cent of the normal amount of clotting factor has **mild** hemophilia.
- A person (male or female) with between 1-5 per cent of the normal amount of clotting factor has **moderate** hemophilia.
- A person (male or female) with less than 1 per cent of the normal amount of clotting factor has **severe** hemophilia.
- A woman who has ≤40% of the normal level of clotting factor would be considered a person with hemophilia. A woman with more than 40% FVIII is considered a carrier and should not be included in this report.

Type of hemophilia	Mild (factor level above 5%)	Moderate (factor level 1% to 5%)	Severe (factor level below 1%)	Severity unknown	No Data
Hemophilia A male	335	67	243	291	☐
Hemophilia A female	0	0	0	0	☐
Hemophilia B male	29	30	39	43	☐
Hemophilia B female	0	0	0	0	☐

The sum of Hemophilia A Male mild, moderate, severe and unknown should be equal to number of Hemophilia A Male in question 6
The sum of Hemophilia A Female mild, moderate, severe and unknown should be equal to number of Hemophilia A female in question 6
The sum of Hemophilia B Male mild, moderate, severe and unknown should be equal to number of Hemophilia B Male in question 6
The sum of Hemophilia B Female mild, moderate, severe and unknown should be equal to number of Hemophilia B female in question 6

Do you consider these numbers to be accurate?	Yes ☒	Not sure ☐

9. Number of severe VWD patients

Total number of severe (type 3) VWD patients	Number of VWD patients receiving replacement therapy	Number of VWD patients with severe bleeding symptoms	No Data
22	130	33	☐

Do you consider these numbers to be accurate?	Yes ☐	Not sure ☒

10. INHIBITORS: Number of identified people with hemophilia with current clinically significant inhibitors in 2017. (Patients who do not respond to normal treatment.)

Type of hemophilia	Total number with active inhibitors	New cases of inhibitors in 2017	No Data
Hemophilia A	22	2	☐
Hemophilia B	2	0	☐

Please Click Here **to validate classification, severity and inhibitors**

11 A. Availability and usage of products to treat hemophilia

Treatment product	Product is available	Product is used	Number of patients treated with product indicated	No data
Plasma	☒	☐		☐
Cryoprecipitate	☒	☐		☐
Plasma-derived concentrate	☒	☒	348	☐
Recombinant concentrate (excluding extended half-life)	☒	☒	262	☐
Recombinant concentrate, extended half-life	☒	☒	10	☐
DDAVP (Desmopressin)	☒	☒		☒

PLEASE NOTE: We are asking for the number of patients treated, not a percentage. Please provide your best estimate.

11 B. Availability and usage of non-factor products to treat hemophilia with inhibitors

Treatment product	Product is available	Product is used	Number of patients treated with product indicated	No data
Hemlibra (Emicizumab)	☐	☐		☐

PLEASE NOTE: We are asking for the number of patients treated, not a percentage. Please provide your best estimate.

12. Availability and usage of products to treat VWD

Treatment product	Product is available	Product is used	Number of patients treated with product indicated	No data
Plasma	☒	☐		☐
Cryoprecipitate	☒	☐		☐
Plasma-derived concentrate	☒	☒	120	☐
DDAVP (Desmopressin)	☒	☒	3	☐

PLEASE NOTE: We are asking for the number of patients treated, not a percentage. Please provide your best estimate.

13. HIV infection

	Hemophilia A or B, or type unknown	von Willebrand disease	Other hereditary bleeding disorders
Total number of people living with HIV	3	0	0
New HIV infections in 2017	0	0	0

14. Hepatitis C infection

	Hemophilia A or B, or type unknown	von Willebrand disease	Other hereditary bleeding disorders
Total number of people infected with hepatitis C[1]	207	3	0
Total number of people with currently active hepatitis C[2]	66	2	0
New hepatitis C infections in 2017	0	0	0

[1]Hepatitis C antibody positive at any time

[2]Still PCR positive: patients who have not cleared the virus spontaneously or after treatment

15. Number and cause of deaths of people with bleeding disorders (January 1-December 31, 2017)

Cause of death	Number of people with Hemophilia A & B	Number of people with von Willebrand disease	Number of people with other inherited bleeding disorders
Bleeding	1	0	0
HIV	0	0	0
Liver disease	0	0	0
Other causes	3	0	0

Please Click Here **to validate products, HIV, HCV, and cause of death sections**

C. Hemophilia Care System in Your Country

We define as Hemophilia Treatment Centre (HTC) a medical centre providing any level of care (including basic diagnosis and treatment) for inherited bleeding disorders. Please provide the number of all such centres in your country. Please also indicate how many of those centers have **direct access, within the same structure,** to at least the following: hemophilia doctor, nurse, physiotherapist, social worker, and special coagulation laboratory.

16. How many **hemophilia treatment centres** are there in total in your country?	11
How many of the **hemophilia treatment centres** you have indicated above have **direct access, within the same structure,** to a hemophilia doctor, nurse, physiotherapist, social worker, and special coagulation laboratory?	11
Which percentage of the hemophilia patients in your country has access to a hemophilia treatment centre:	100

Prophylaxis is regular, long-term treatment with clotting factor concentrates to prevent bleeds. Please indicate if the percentage provided is precise or an estimate.

17. What percentage of children (18 and under) **with severe hemophilia** are on prophylaxis?	93	Precise: ☒ Estimate: ☐	Not known ☐
What percentage of adults (over age 18), **with severe hemophilia** are on prophylaxis?	58	Precise: ☒ Estimate: ☐	Not known ☐
What is the most common dose (IU/kg) of factor administered and frequency?	Prophy adults:18 IU/kg 3/week, kids 25 IU/kg 3/week		

Immune tolerance induction (ITI) is the administration of FVIII or FIX concentrate in patients with inhibitors to eradicate the inhibitors. Please indicate the total # of patients with inhibitors who received ITI in your country in the last year, and the number of new patients who started ITI during the last year. Please indicate if these #s are precise or an estimate.

18. What is the total number of patients **with inhibitors** who received ITI during the last year?	7	Precise: ☒ Estimate: ☐	Not known ☐
Of this total, how many were new patients who **started** ITI treatment during the last year?	3	Precise: ☒ Estimate: ☐	Not known ☐

Please Click Here **to validate Care section**

D. The Cost and Use of Factor Concentrates

19 A. Annual usage of purchased factor concentrates (please do not include donated factor)	Factor VIII	Not known	Factor IX	Not known
IN TOTAL how many international units (IU) of factor concentrates were used in your country in 2017 (excluding donated factor)?	65722514	☐	7411857	☐
Plasma derived: How many international units of **plasma-derived** concentrates were used in your country in 2017 (excluding donated factor)?	29051202	☐	4841771	☐
Recombinant, excluding extended half-life: How many international units of **recombinant** concentrates **(excluding extended half-life)** were used in your country in 2017 (excluding donated factor)?	34856912	☐	2065960	☐
Recombinant, extended half-life: How many international units of **recombinant** concentrates**, extended half-life** were used in your country in 2017 (excluding donated factor)?	1814400	☐	504126	☐
If factor concentrates are purchased in your country but you are unable to report the quantities please check here:	☐		☐	

The Total of FVIII should be equal to sum of FVIII plasma-derived and FVIII recombinant
The Total of FIX should be equal to sum of FIX plasma-derived and FIX recombinant

19 B. Annual usage of donated factor concentrates	Factor VIII	Not known	Factor IX	Not known
How many international units of **donated factor** concentrates (plasma-derived or recombinant) from all sources, including **Humanitarian Aid,** were used in your country in 2017?	0	☐	0	☐

Do you consider these numbers to be accurate?	Yes ☒	Not sure ☐

PLEASE NOTE: If a product used in your country is not listed, please add it at the bottom of the appropriate table.

Currency: CZK	Tax included? No ☐ Yes ☒	Tax rate:

Please Click Here **to validate Factors section**

20. Factor VIII Concentrates used in 2017

(Please check the box on the left if a product is used, and if known, fill out the cost per international unit in the currency used to purchase the product. Please indicate if this price includes tax.)

Used	Brand Name	Manufacturer	Price per IU
☐	Aafact	Sanquin	
☒	Advate rAHF PFM	Baxalta (now part of Shire)	
☐	Adynovate	Baxalta (now part of Shire)	
☐	Aleviate	CSL Behring	
☐	Alphanate	Grifols	
☐	Amofil	Sanquin OY	
☐	Bioclot A	Biofarma	
☐	Beriate P	CSL Behring	
☐	BIOSTATE	CSL Bioplasma	
☐	Conco-eight-HT	Benesis	
☐	Confact F	Kaketsuken	
☐	Cross Eight M	Japanese Red Cross	
☒	Elocta/Eloctate	Biogen Idec	
☐	Emoclot D.I.	Kedrion	
☐	FACTANE	LFB	
☐	Factor 8 Y	BioProducts Lab.	
☐	Faktor VIII SDH Intersero	Intersero	
☒	Fanhdi	Grifols	
☐	GreenEight	GreenCross	
☐	GreenGene	GreenCross	
☐	GreenMono	Greencross Corp	
☒	Haemate P (= Haemate HS)	CSL Behring	
☒	Haemoctin SDH	Biotest	
☐	Haemosolvate Factor VIII	National Bioproducts	
☐	Helixate NexGen = Helixate FS	CSL Behring	
☐	HEMO-8R	HEMOBRAS	
☐	Hemofil M AHF	Baxalta (Baxter Bioscience)	
☐	HEMORAAS SD plus H	Shanghai RAAS	
☐	HEMORAAS-HP, SD plus H	Shanghai RAAS	
☐	HEMORAAS-IP, SD plus H	Shanghai RAAS	
☐	Humate P	CSL Behring	
☐	Humafaktor 8	Human BioPlazma	
☐	Human Coagulation Factor VIII	Baltijas Terapeitiskais Serviss	
☒	Immunate	Baxalta (now part of Shire)	
☐	Koate DVI	Talecris	

☒	Kogenate FS = KOGENATE Bayer (in EU)	Bayer	
☐	Monoclate P	CSL Behring	
☒	Novoeight	NovoNordisk	
☒	Nuwiq	Octapharma	
☒	Octanate	Octapharma	
☐	Octanativ-M	Octapharma	
☐	Octavi SD	Octapharma	
☐	Octofactor	Generium/Pharmstandart	
☐	Optivate	Bio Products Laboratory	
☐	FVIII by Quimbiotec	Quimbiotec	
☒	Recombinate rAHF	Baxalta (now part of Shire)	
☒	ReFacto AF	Pfizer (Wyeth)	
☐	Replenate	Bio Products Laboratory	
☐	TBSF purity factor, Koate DVI	Grifols	
☐	UNC Hemoderivados	Laboratorio de Hemoderivados de Universidad Nacional de Córdoba	
☐	Voncento	CSL Behring	
☐	Western Province factor8 VIAHF	Western Province Blood transfusion Service	
☒	Wilate	Octapharma	
☐	Xyntha	Pfizer (Wyeth)	
☐	Other:		

PLEASE NOTE: For "Other", please provide the Brand Name and Manufacturer.

21. Factor IX Concentrates used in 2017

(Please check the box on the left if a product is used, and if known, fill out the cost per international unit in your currency.)

Used	Brand Name	Manufacturer	Price per IU
☐	Aimafix	Kedrion	
☐	AlphaNine SD	Grifols	
☒	Alprolix	Biogen Idec	
☒	BeneFIX	Wyeth	
☐	Berinin-P = Berinin HS	CSL Behring	
☐	BETAFACT	LFB	
☐	Christmassin-M	Benesis	
☐	Clotnine	Hemarus	
☐	Factor IX Grifols	Grifols	
☐	Faktor IX SDN	Biotest	
☐	Fixnove	Baxalta (now part of Shire)	
☐	Hemo-B-RAAS	Shanghai RAAS	
☐	Haemonine	Biotest	
☐	Humafactor IX	Kedrion	
☒	Immunine	Baxalta (now part of Shire)	

☐	MonoFIX-VF	CSL Bioplasma	
☒	Mononine	CSL Behring	
☐	Nanofix	Octapharma	
☐	Nanotiv	Octapharma	
☐	Nonafact	Sanquin	
☐	Novact M	Kaketsuken	
☐	Octafix	Octapharma	
☒	Octanine F	Octapharma	
☐	Replenine – VF	BioProducts Lab.	
☒	Rixubis	Baxalta (now part of Shire)	
☐	Other:		

PLEASE NOTE: For "Other", please provide the Brand Name and Manufacturer.

22. Prothrombin Complex Concentrates used in 2017

(Please check the box on the left if a product is used, and if known, fill out the cost per international unit in your currency.)

Used	Brand Name	Manufacturer	Price per IU
☐	Bebulin VH	Baxalta (now part of Shire)	
☒	Beriplex P/N	CSL Behring	
☐	Cofact	Sanquin	
☐	Facnyne	Greencross Corp	
☐	Haemosolvex Factor IX	National Bioproducts	
☐	HT DEFIX	SNBTS	
☐	Kanokad Confidex	LFB	
☐	KASKADIL	LFB	
☒	Octaplex	Octapharma	
☐	PPSB-HT	Nihon Pharmaceutical	
☐	PPSB-human SD/Nano 300/600	German Red Cross NSTOB	
☐	Profilnine SD	Grifols	
☒	Proplex – T	Baxalta (now part of Shire)	
☐	Prothrombinex PXT	CSL Bioplasma	
☐	Prothrombinex- VF	CSL Bioplasma	
☒	Prothromplex-T	Baxalta (now part of Shire)	
☐	Prothroraas	Shanghai RAAS	
☐	UMAN Complex D.I.	Kedrion	
☐	Other:		

PLEASE NOTE: For "Other", please provide the Brand Name and Manufacturer.

23. Other Products used in 2017

(Please check the box on the left if a product is used, and if known, fill out the cost per international unit in your currency.)

Used	Brand Name	Manufacturer	Price per IU
☐	Aryoseven	Aryogen	

☐	Byclot (1.5mg)	Kaketusken	
☒	Ceprotin	Baxalta (now part of Shire)	
☐	Clottafact Wilstart	LFB	
☐	Clottagen (fibrinogen)	LFB	
☐	Coagil 7 (activated factor VII)	Pharmstandard	Price per vial: Vial size:
☐	FACTEUR VII	LFB	
☒	Factor VII	Baxalta (now part of Shire)	
☐	Factor VII	Bio Products	
☐	Factor X P Behring	CSL Behring	
☐	Factor XI	Bio Products	
☒	FEIBA	Baxalta (now part of Shire)	
☐	Fibrinogen HT	Benesis	
☐	Fibrogammin P (=Fibrogammin HS) (Factor XIII)	CSL Behring	
☐	FIBRORAAS (fibrinogen)	Shanghai RAAS	
☒	Haemocomplettan P = Haemocomplettan HS (fibrinogen)	CSL Behring	
☒	HEMOLEVEN (Factor XI)	LFB	
☒	Kovaltry	Bayer	
☒	NovoSeven (=Niastase) (activated factor VII)	NovoNordisk	Price per vial: Vial size:
☐	Riastap	CSL Behring	
☐	Tretten rXIII	NovoNordisk	
☒	WILFACTIN (Von Willebrand Factor)	LFB	
☐	Other:		

PLEASE NOTE: For "Other", please provide the Brand Name and Manufacturer.

24. Non-factor products used in 2017

(Please check the box on the left if a product is used, and if known, fill out the number of patients and price per dose.)

Used	Brand Name	Manufacturer	Price per Dose
☐	Hemlibra (Emicizumab)	Roche	

Please return to:

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1425 René Lévesque Boulevard West, suite 1200
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Please provide your feedback on the WFH Annual Global Survey data collection system.

Comments:

Glossary of terms

Bernard-Soulier syndrome: A severe congenital bleeding disorder characterized by thrombocytopenia and large platelets, due to a defect in the platelet glycoprotein 1b/V/IX receptor.

Cryoprecipitate: A fraction of human blood prepared from fresh plasma. Cryoprecipitate is rich in factor VIII, von Willebrand factor, and fibrinogen (factor I). It does not contain factor IX.

Desmopressin (DDAVP): A synthetic hormone used to treat most mild cases of von Willebrand disease and mild hemophilia A. It is administered intravenously or by subcutaneous injection or by intranasal spray.

Factor concentrates: These are fractionated, freeze-dried preparations of individual clotting factors or groups of factors derived from donated blood.

Glanzmann's thrombasthenia: A severe congenital bleeding disorder in which the platelets lack glycoprotein IIb/IIIa, the blood platelet count is normal, but their function is very abnormal.

Hemophilia A: A condition resulting from factor VIII deficiency, also known as classical hemophilia.

Hemophilia B: A condition resulting from factor IX deficiency, also known as Christmas disease.

Hemophilia treatment centre: A specialized medical centre that provides diagnosis, treatment, and care for people with hemophilia and other inherited bleeding disorders.

HIV: Human immunodeficiency virus. The virus that causes AIDS.

Identified person: A living person known to have hemophilia, von Willebrand disease, or another bleeding disorder.

Inhibitors: A PWH has inhibitors when their body's immune system attacks the molecules in factor concentrate, rendering it ineffective.

International Unit (IU): A standardized measurement of the amount of factor VIII or IX contained in a vial. Usually marked on vials as 250 IU, 500 IU, 1000 IU or 2000 IU.

Mild hemophilia: Condition resulting from a level of factor VIII or factor IX clotting activity below normal but above 5% of normal activity in the bloodstream. (National definitions differ on the upper limit for mild hemophilia, ranging from 24% to 50%. The normal range of factor VIII or IX is 50 to 200%)

Moderate hemophilia: Condition resulting from a level of factor VIII or factor IX clotting activity between 1 to 5 % of normal activity in the bloodstream.

Plasma-derived products: Factor concentrates that contain factor VIII or IX that have been fractionated from human blood.

PWH: Person with hemophilia

Recombinant products: Factor concentrates that contain factor VIII or IX that have been artificially produced and are, therefore, not derived from human blood.

Registry: A database or record of identified people with hemophilia or inherited bleeding disorders. A registry includes information on personal details, diagnosis, treatment and complications.

Severe hemophilia: Condition resulting from a level of factor VIII or factor IX clotting activity of less than 1 % in the bloodstream.

von Willebrand disease (VWD): An inherited bleeding disorder resulting from a defect or deficiency of von Willebrand factor.