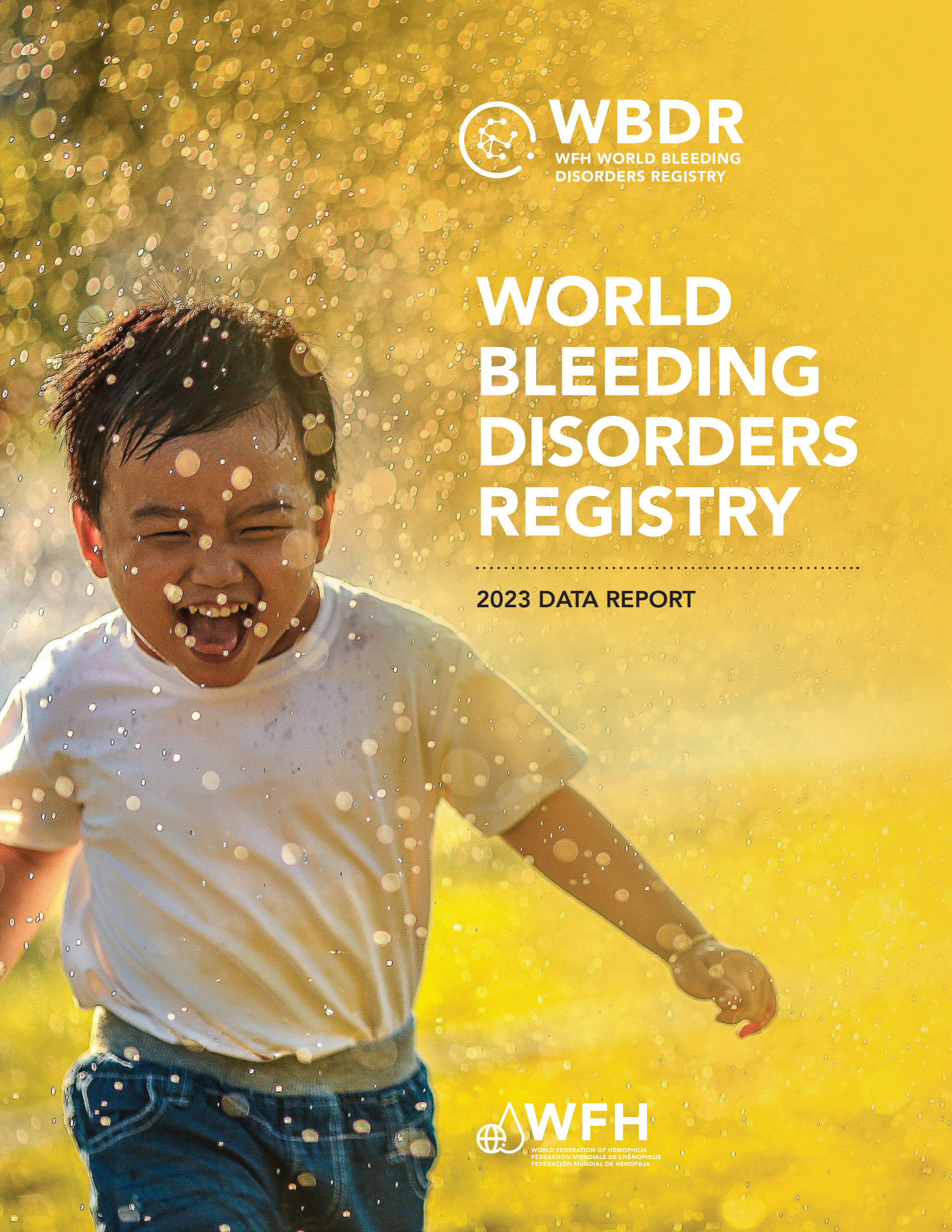


WBDR
WFH WORLD BLEEDING DISORDERS REGISTRY

WORLD BLEEDING DISORDERS REGISTRY

2023 DATA REPORT

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

TABLE OF CONTENTS

ABOUT THE WFH

For over 60 years, the WFH – an international not-for-profit organization – has worked to improve the lives of people with hemophilia, von Willebrand disease (VWD) and other inherited bleeding disorders. Established in 1963, it is a global network of patient organizations in 147 countries and has official recognition from the World Health Organization (WHO). To find out more about the WFH, visit www.wfh.org.

MISSION OF THE WORLD FEDERATION OF HEMOPHILIA

IMPROVE AND SUSTAIN CARE FOR PEOPLE WITH INHERITED BLEEDING DISORDERS AROUND THE WORLD.

MISSION

ACKNOWLEDGEMENTS

WFH Research & Education department who contributed to the creation of this report:

- Donna Coffin, MSc
- Emily Ayoub, PhD
- Ellia Tootoonchian, MPH
- Pamela Dakik, PhD
- Toong Youttananukorn, PhD

Special thanks to Ticiana Carvalho Pereira for her contribution to the statistical analysis of the WBDR data.

PRESIDENT & VP MEDICAL'S MESSAGE

March 2024

Dear members of the bleeding disorders community,

We are delighted to present to you the 2023 World Bleeding Disorders Registry (WBDR) Data Report. This report marks the sixth year of our global effort to systematically capture the real-world clinical experience of people with hemophilia (PWH). Additionally, it signifies the inaugural year of collecting data on people with von Willebrand disease (PwVWD) from around the world. It is our hope that these data will support research and advocacy initiatives, serve as a valuable tool for clinical decision making, and improve the overall care for people with bleeding disorders.

As of December 31, 2023, over 13,000 PWH from 119 hemophilia treatment centres (HTCs) and 44 countries around the world have joined our efforts in collecting these valuable data. In this report you will find the aggregated summary of data for all PWH, as well as those with VWD. This year marked the launch of WBDR's data collection for PwVWD. There have been 45 HTCs from 22 countries who have enrolled nearly 1,000 PwVWD since the beginning of the year.

WBDR has made significant strides in terms of participation and patient enrollment, with programs such as the Research Support Program and HTC Funding Program serving as major catalysts for enhancing data collection capabilities.

On behalf of the WFH, we would like to warmly thank all of the dedicated health care providers, PWH and PwVWD who are part of this important initiative. We look forward to further collaborations with HTCs interested in participating in the WBDR as WFH pushes towards its mission of Treatment for All.

We would also like to recognize our visionary partners who have made it possible for us to develop this registry: Sobi and Takeda; as well as our collaborating partners: F. Hoffmann-La Roche, Grifols, Novo Nordisk, Pfizer & Sanofi

Sincerely,

Cesar Garrido
President

Glenn Pierce
VP Medical

ABOUT THE WBDR

Launched in January 2018, the WFH WBDR provides a platform for HTCs around the world to collect standardized data on people with hemophilia and VWD. The WBDR is a prospective, longitudinal, observational registry. It is a privacy-protected online web-based data entry system, that allows for the collection of individual patient data, thus providing a clinical profile for each patient.

THE WBDR IS OPEN TO
ALL PEOPLE WITH HEMOPHILIA A OR B, AND VWD WHO ARE PATIENTS AT A PARTICIPATING HTC.

WBDR METHODOLOGY

Participating HTCs are at the forefront of recruiting patients and entering confidential and de-identified patient data into the WBDR database. The WFH works closely with all interested HTCs to guide and assist them through the required steps of participating in the program, including obtaining Institutional Review Board approval, recruiting patients, and managing their data.

The WBDR is open to all people with hemophilia A or B and PwVWD who are a patient at a participating HTC. The HTCs are asked to invite all consecutive people with hemophilia A and B, and VWD at their clinic to enroll in the WBDR to minimize the risk of selection bias. All patients who agree to participate must provide informed consent.

REPORT DATA SOURCE

The data presented in the WBDR 2023 Data Report include aggregate and de-identified data from patients who received care at a participating hemophilia treatment centre (HTC) and who consented to have their data entered into the World Bleeding Disorders Registry (WBDR).

IMPLEMENTATION

Implementation of the WBDR begins with the HTCs. Candidate HTCs are identified, with the help of our National Member Organizations (NMO), and invited to register with the WBDR, directly by the WBDR team. Interested HTCs can contact the WBDR team at wbdr@wfh.org. The WBDR team is available to assist HTCs in obtaining ethical approval from their local organization.

INSTITUTIONAL REVIEW BOARDS/ETHICS COMMITTEE

Hemophilia treatment centres must obtain Institutional Research Board or Ethics Committee approval from their local institution prior to enrolling patients into the WBDR. All WBDR documents required for ethics submission are provided to HTCs, and translated versions are available upon request.

INFORMED CONSENT

People with hemophilia and VWD who are interested in participating in the WBDR must be a patient at participating HTC and must provide informed consent to have their confidential and de-identified data entered into the registry. If a patient decides not to participate, they will continue to receive the same care as all other patients at their HTC. For patients who decide to participate in the WBDR, the treatment team of the HTC will record patient data after each clinic visit and enter it into the WBDR.

COLLECTION OF DATA AND FOLLOW-UP VISITS

Patient data are collected at the baseline visit (the visit at which patients provide informed consent) and at all subsequent follow-up clinic visits. At the baseline visit, retrospective data based on the previous six months are collected. At each subsequent follow-up visit, data for the period since the previous clinic visit are collected. This method ensures that all data and events over the course of time are captured.

WBDR DATA

At the time of the launch of the WBDR in 2018, a minimal data set was introduced. In February 2019, an extended data set (EDS) was developed and implemented. The data in this report are based on both minimal and extended data sets (Appendix 1).

UNIQUE PATIENT IDENTIFIER

Using a cryptographic hashing process, all patients entered into the WBDR are provided a unique patient identifier (UPI). The UPI reduces the risk of duplicate patients being entered into the WBDR and will be useful for linking with other databases in the future. For more information on the UPI and the cryptographic process, please see the WBDR Data Privacy & Security document.

TRANSFER PATIENTS

Patients can be transferred between participating HTCs within the WBDR. This transfer function is useful in countries where patients receive care at more than one HTC.

INTERNATIONAL DATA INTEGRATION PROGRAM

The WBDR includes an international data integration component, whereby existing hemophilia registries can import their data directly into the WBDR and become part of this international registry.

Please see page 44 for more information.

DATA QUALITY

The WBDR Data Quality Accreditation program is designed to enhance the completeness, accuracy and consistency of the data entered in the WBDR. The WBDR team works closely with all HTCs to ensure their data meets the WBDR data quality standards.

Please see page 42 for more information on the WBDR data quality program.

HTC SUPPORT AND TRAINING PROGRAMS

The WBDR support and training programs are available to all participating HTCs, including the Research Support Program and the HTC Funding Program. These programs were developed to ensure long-term success in the WBDR. In-person and webinar trainings are available on:

- Ethics submission process
- Obtaining informed consent
- Data entry
- Data quality management
- Using data effectively for research and advocacy purposes

DATA ACCESS AND GOVERNANCE

Each HTC has access only to the data they enter into the WBDR, and they cannot view data that are entered from any other HTC. Every year, aggregate data from all enrolling HTCs are published in the WBDR Data Report. Access to data for research and advocacy purposes will be available through the WBDR Research Governance Committee.

DATA PRIVACY

The WBDR database was developed through the collaborative efforts of the WFH, the Karolinska Institute, and BCB Medical – the latter two organizations based in Sweden. All patient information entered in the WBDR are de-identified and confidential. Data policy guidelines of BCB Medical adhere to the CE-mark (Conformité Européenne) and the U.K. standard IG SoC (Information Governance Statement of Compliance) and are compliant with the General Data Protection Regulation. Please see the WBDR Data Privacy & Security document for more information.

ABOUT THE WBDR 2023 DATA REPORT

The data in this edition of the WBDR Data Report includes patient data collected between the launch date of January 26, 2018, and December 31, 2023. These data stem from 119 participating HTCs (Appendix 2), representing 44 countries, who received institutional review board approval and enrolled at least one patient into the WBDR as of December 31, 2023. The report is separated into two sections. First section focuses on people with hemophilia, while the second highlights the first year of data collection on PwVWD.

Please note that at the time of data cut-off for this report (December 31, 2023), it is possible that not all eligible patients at participating HTCs had been enrolled into the WBDR. Therefore, the data in this report may not represent the entire patient population at each HTC, limiting generalizability. As the proportion of patients enrolled in the WBDR at participating HTCs increases, the data will become more reflective of the patient population at each HTC.

The 2023 WBDR data are reported using frequency distributions and percentages for categorical data, and medians with quartiles 1 and 3, denoted as (Q1 – Q3), and/or range, for continuous variables.

WFH WBDR STEERING COMMITTEE

The WFH would like to thank the current WBDR Steering Committee for their dedication to the development and implementation of the WBDR:

- Alfonso Iorio, MD, PhD, Co-Chair
- Emna Gouider, MD, Co-Chair
- Barbara Konkle, MD
- Saliou Diop, MD
- Cedric Hermans, MD, PhD
- Catherine Lambert, MD
- Jamie O'Hara, MSc
- Glenn Pierce, MD, PhD, VP Medical WFH
- Cesar Garrido, President WFH

GLOBAL REPRESENTATION IN THE WBDR, 2023

14,342 PWH and PwVWD have been enrolled in the WBDR

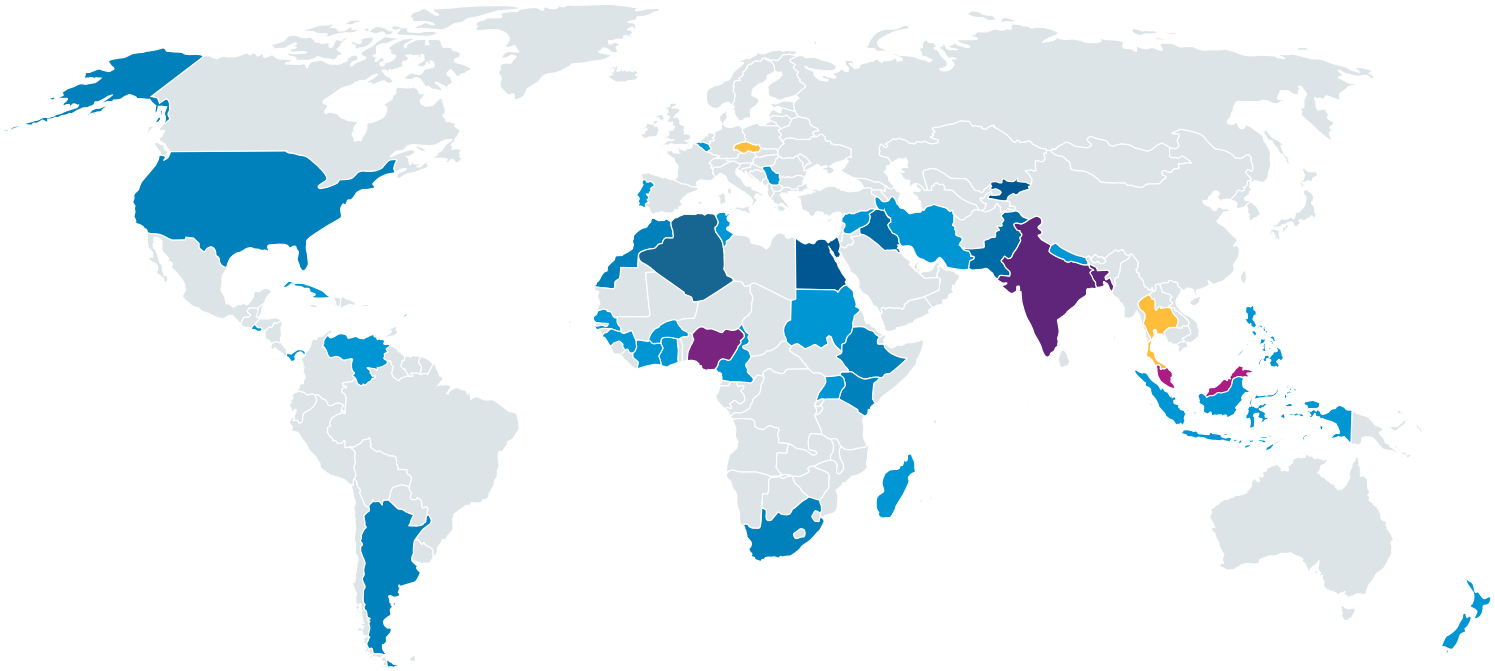


Figure 1
COUNTRIES AND HTCS PARTICIPATING IN THE WBDR

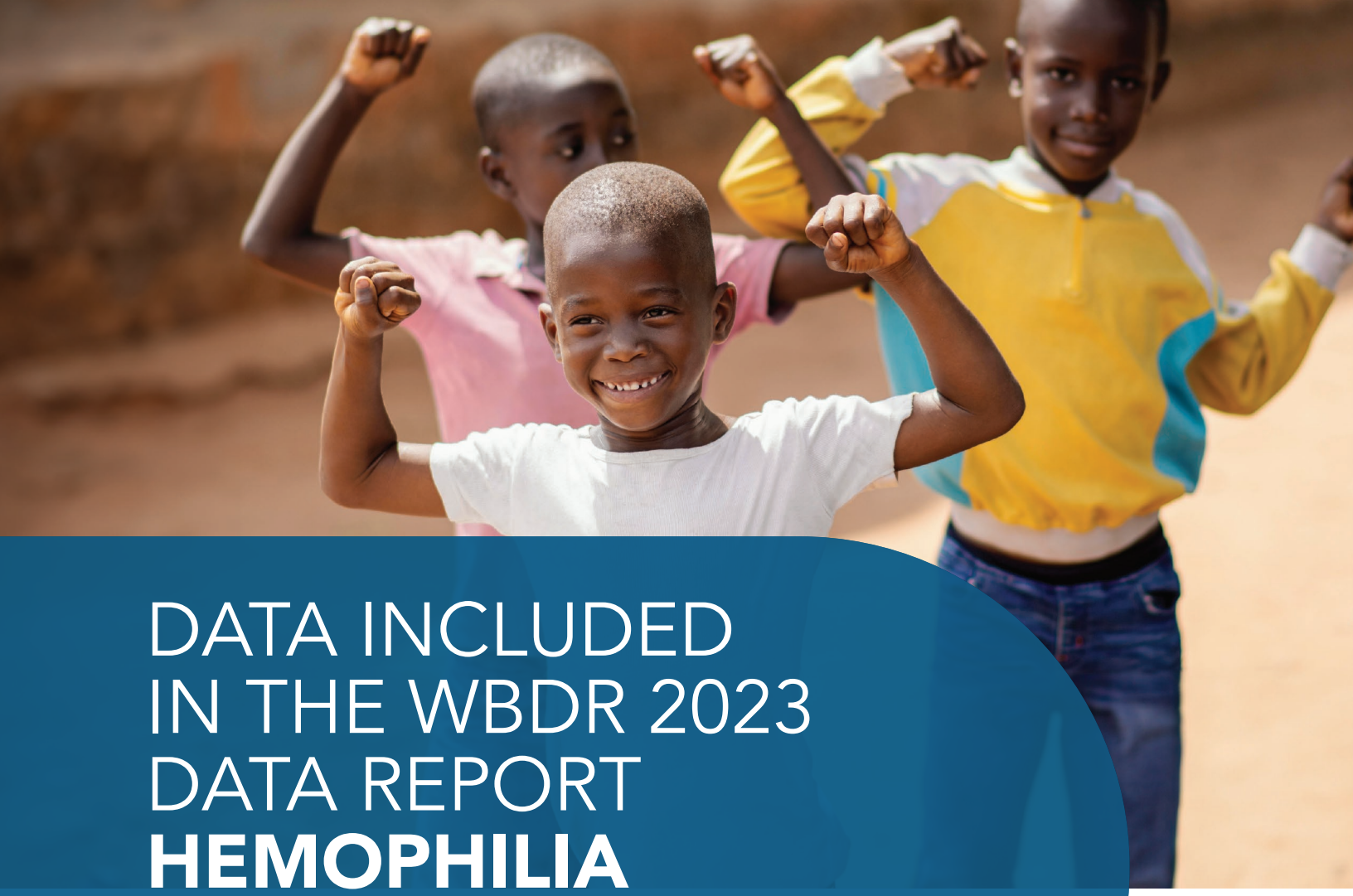
Number of HTCs per country

1 — 17

Data Integration

For a complete list of HTCs, please refer to Appendix 2

Algeria	2	Madagascar	1
Argentina	2	Malaysia	15
Bangladesh	6	Morocco	2
Barbados	1	Nepal	1
Belgium	1	New Zealand	2
Burkina Faso	1	Nigeria	7
Cameroon	1	Pakistan	3
Cuba	1	Panama	1
Czechia	17	Philippines	1
Egypt	4	Portugal	1
El Salvador	1	Senegal	1
Ethiopia	1	Serbia	1
Ghana	1	South Africa	2
Guinea	1	Sudan	1
India	6	Syria	1
Indonesia	1	Thailand	8
Iran	1	Tunisia	1
Iraq	3	Uganda	1
Ivory Coast	1	USA	2
Kenya	2	Venezuela	1
Kyrgyzstan	4		



DATA INCLUDED IN THE WBDR 2023 DATA REPORT **HEMOPHILIA**

119
HTCs ENROLLED

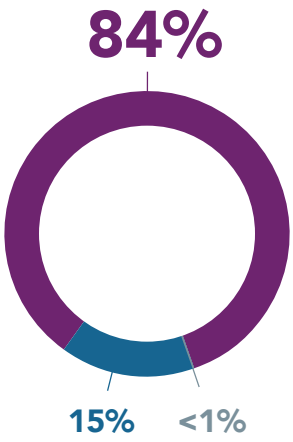
44
COUNTRIES REPRESENTED

13,343
ENROLLED PATIENTS

11,245
Hemophilia A

2,050
Hemophilia B

48
Unknown



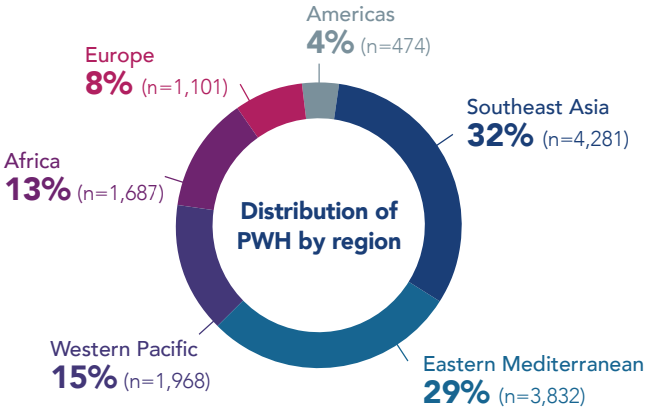


TABLE 1

Hemophilia Participation Summary

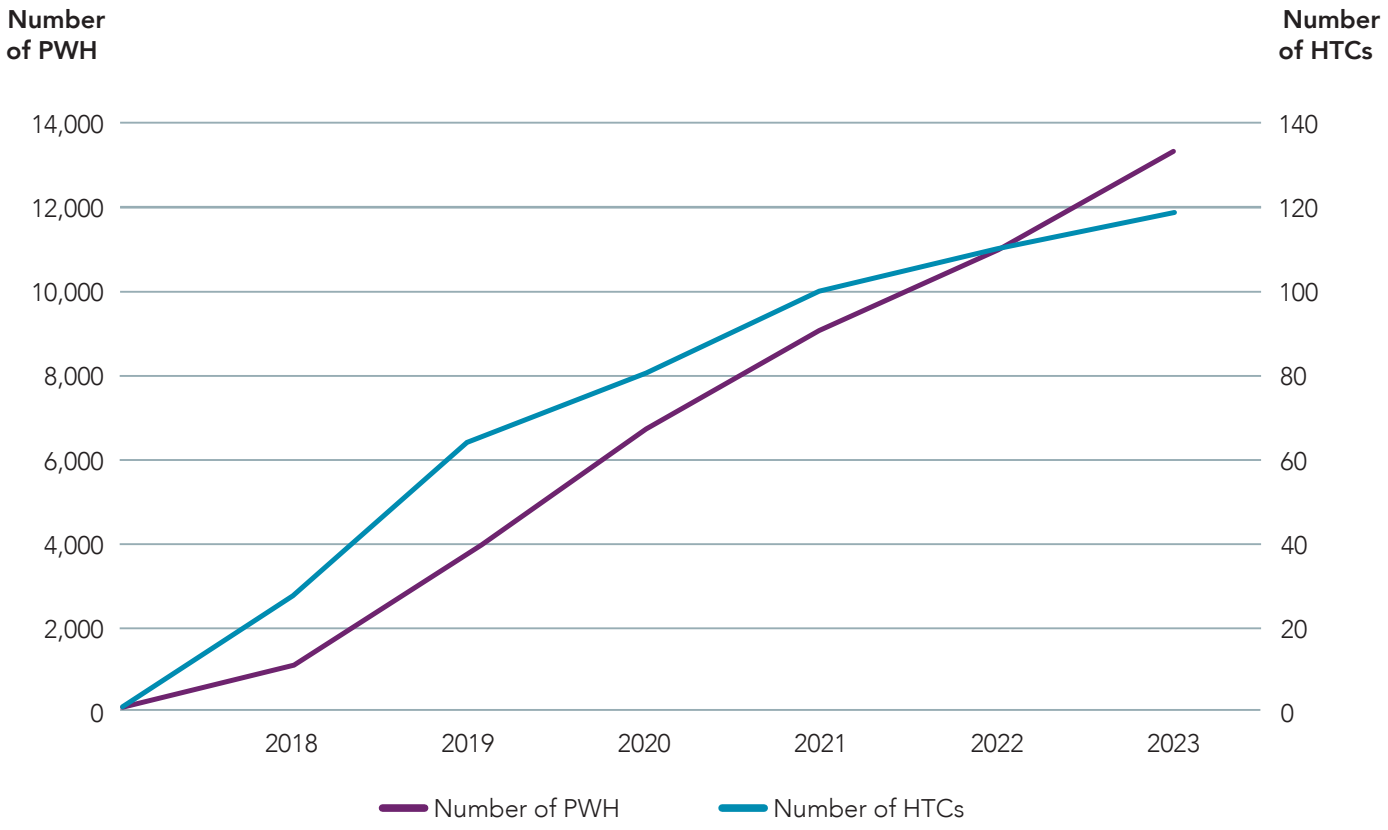
	Mild & Moderate PWH*	Severe PWH*
Countries, n	43	43
Hemophilia treatment centres**, n	103	113
People with hemophilia, n	6,194	6,616
Distribution of PWH by region†, n (%)		
Africa (n=1,687)	473 (28%)	796 (47%)
Americas (n=474)	125 (26%)	340 (72%)
Eastern Mediterranean (n=3,832)	1,782 (47%)	1,989 (52%)
Europe (n=1,101)	614 (56%)	475 (43%)
Southeast Asia (n=4,281)	2,390 (56%)	1,861 (43%)
Western Pacific (n=1,968)	810 (41%)	1,155 (59%)
Distribution of PWH by GNI§, n (%)		
High income (n= 1,309)	606 (54%)	501 (45%)
Upper-middle income (n=1,945)	709 (34%)	1,356 (65%)
Lower-middle income (n= 7,575)	4,515 (48%)	4,486 (48%)
Low income (n=545)	366 (47%)	273 (35%)

* Severe PWH are defined by a factor level <1%. 533 PWH with unknown severity were excluded resulting in totals not adding up to 100%.
** HTCs included are those with Institutional Board Review approval and have enrolled at least 1 PWH by December 31, 2023.
† Regions based on WHO regional groupings[2]
§ GNI = Gross National Income; Gross National Income categories based on The World Bank Group 2023 rankings for "Gross national income (GNI) per capita, Atlas method (current US$)"[3].

Figure 2

PWH and HTC enrollment in the WBDR

January 2018 – December 2023

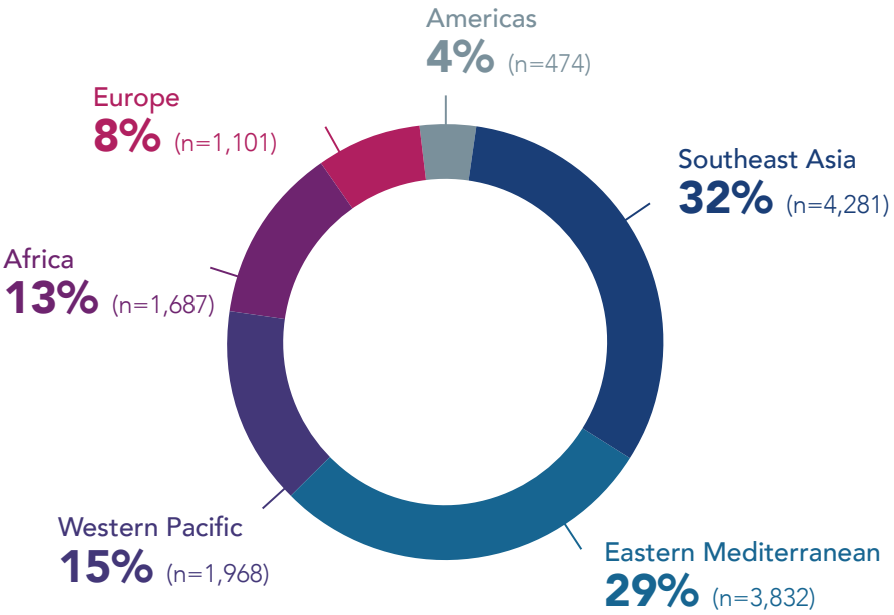


DISTRIBUTION OF PWH

The regional classification used in the WBDR is based on the WHO regional classification[2]. The majority of PWH are from the Southeast Asia region (Bangladesh, India, Indonesia, Nepal, and Thailand) and the Eastern Mediterranean (Algeria, Egypt, Iran, Iraq, Morocco, Pakistan, Sudan, Syria, and Tunisia), representing 32% and 29% of PWH respectively (Figure 3).

Figure 3

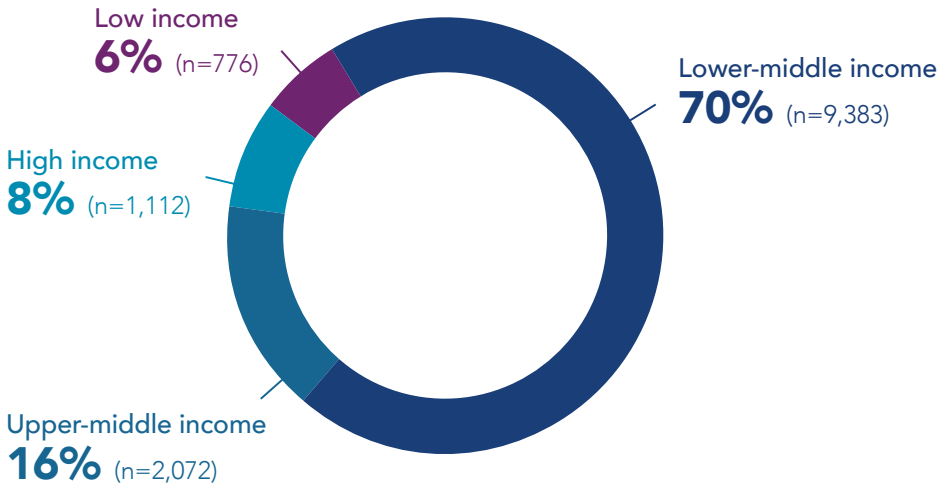
Distribution of PWH by region



The distribution of participants by Gross National Income (GNI) per capita[3], demonstrates that the majority of the participant PWH are from lower-middle income countries (70%), followed by upper-middle, high, and low income representing 16%, 8% and 6% respectively (Figure 4).

Figure 4

Distribution of PWH by Gross National Income



HEMOPHILIA DEMOGRAPHICS

TABLE 2

Hemophilia demographics summary

	Mild & Moderate PWH*	Severe PWH*
Type of hemophilia, n (%)**		
Hemophilia A (n=11,245)	5,117 (46%)	5,692 (51%)
Hemophilia B (n=2,050)	1,068 (52%)	919 (45%)
Sex, n (%)		
Male (n=13,167)	6,074 (46%)	6,574 (50%)
Female (n=174)	119 (68%)	41 (24%)
Age of PWH$		
Age, years, median (IQR)	21 (12-34)	20 (11-32)
Pediatrics (<18 years), (n=5,843)	2,575 (44%)	2,960 (51%)
Adults (≥18 years), (n=7,500)	3,619 (48%)	3,656 (49%)

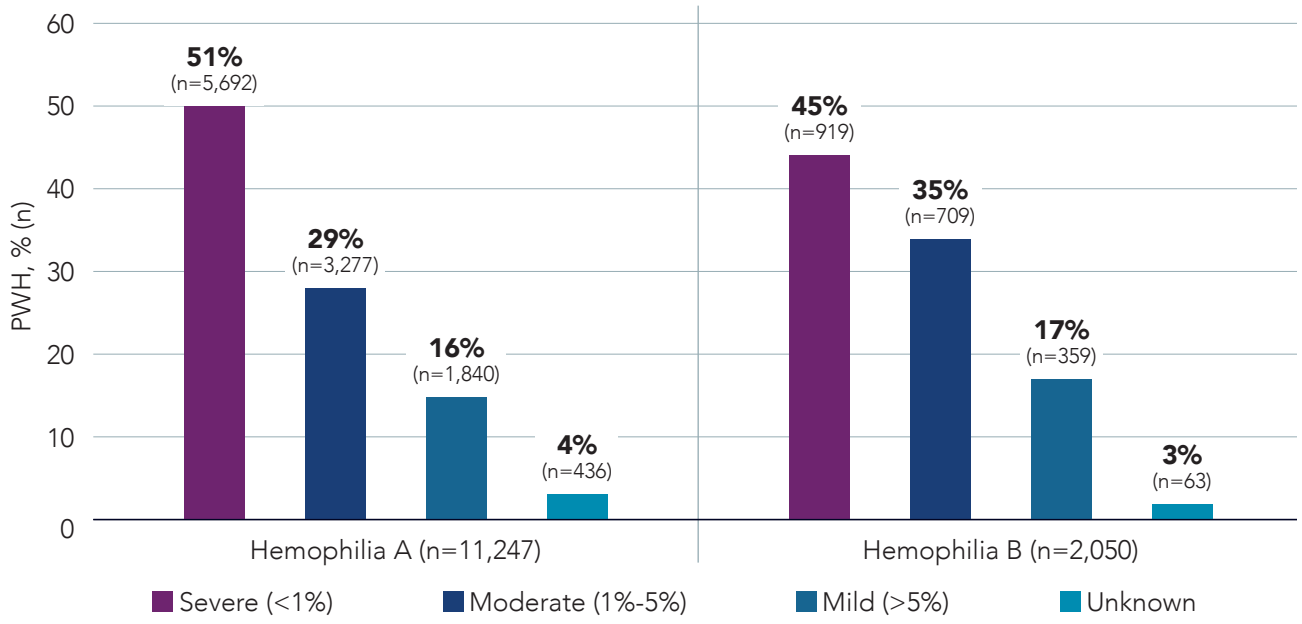
IQR=interquartile range
* 533 PWH with unknown severity were excluded resulting in totals not adding up to 100%.
** 48 PWH had unknown hemophilia type and were excluded resulting in totals not adding up to 100%.
$ Age of PWH was calculated as of December 31, 2023.

HEMOPHILIA TYPE AND SEVERITY

Overall, 99% (n=13,167) of participants were male, 84% (n=11,245) had hemophilia A and 15% (n=2,050) had hemophilia B (Table 2). The most frequent severity category among hemophilia A and hemophilia B patients was severe at 51% and 45% respectively (Figure 5).

Figure 5

Hemophilia type* and severity, % (n)



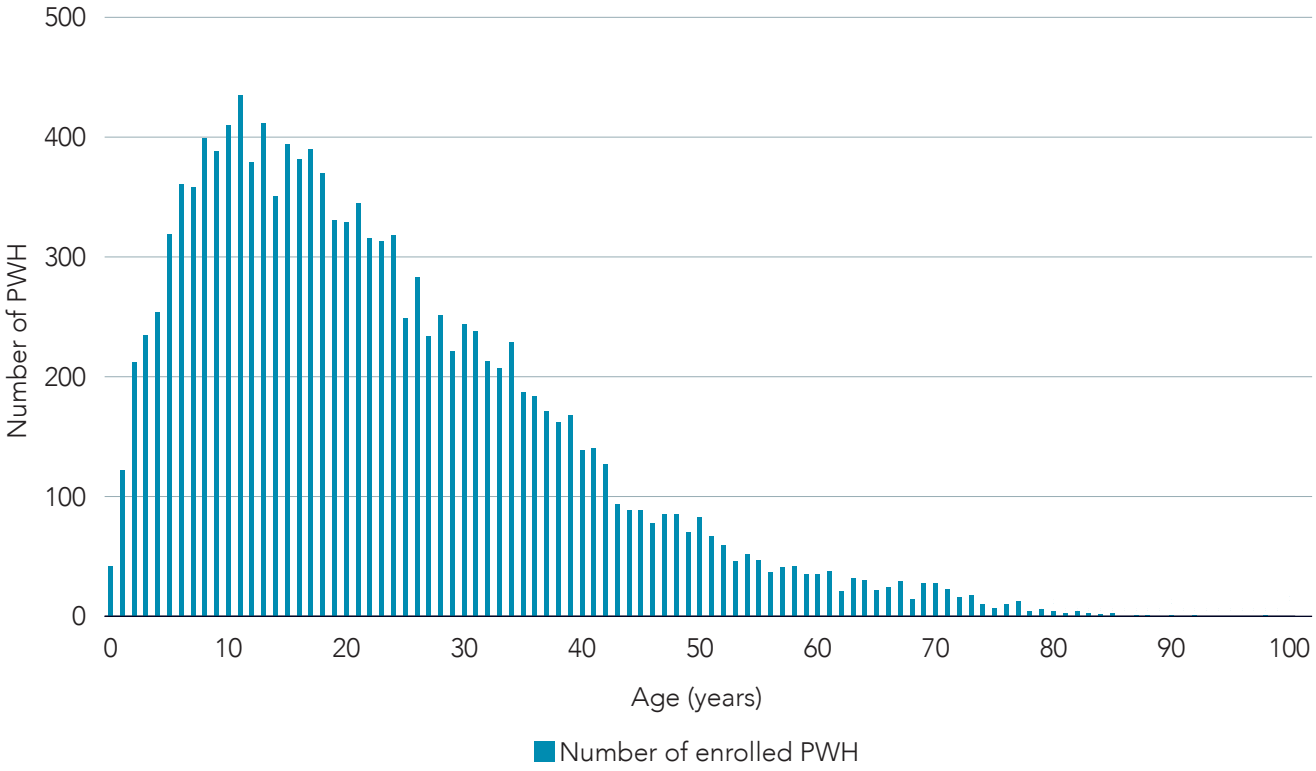
* 48 PWH had unknown hemophilia type and were excluded from this figure.

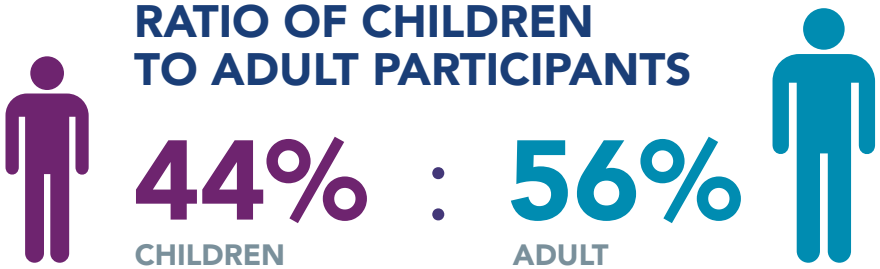
AGE OF PWH IN THE WBDR

The median age of PWH was 20 years, ranging from 1 month to 98 years (Figure 6). Adults (≥18) represent 56% (n=7,500) and children (<18) represent 44% (n=5,843) of all PWH.

Figure 6

Age distribution of PWH in the WBDR





HEMOPHILIA DIAGNOSIS AND CLINICAL HISTORY

TABLE 3a

Diagnosis and clinical history summary

	Mild & moderate PWH (n=6,194)	Severe PWH (n=6,616)
Age at diagnosis, months, median (IQR)	62 (16-172)	20 (7-88)
Age at diagnosis by age category, n (%)		
0–11 months	1198 (19%)	2427 (37%)
1–4 years	1803 (29%)	2044 (31%)
5–17 years	1908 (31%)	1373 (21%)
18–44 years	1006 (16%)	644 (10%)
45+ years	199 (3%)	56 (<1%)
Age unknown	80 (1%)	72 (1%)

TABLE 3b

Newly diagnosed PWH in 2023, n (%)	226 (4%)	149 (2%)
Age at first bleed*, months, median (IQR)	18 (6-60)	8 (5-18)
Age at first joint bleed, months, median (IQR)**	36 (12-74)	18 (10-47)

* Based on 10,408 PWH with data on first bleed.
** Based on 8,418 PWH with data on first joint bleed.

AGE AT DIAGNOSIS

The median (IQR) age at diagnosis was 37 months (10-128) for all PWH, 62 months (16-172) for mild & moderate PWH, and 20 months (7-88) for severe PWH (Table 3a). For mild & moderate PWH, median age at diagnosis by region ranged from 19 months in the Americas to 84 months in Western Pacific (Figure 7). In severe PWH, the highest age at diagnosis was in Africa at 45 months and lowest was the Americas at 8 months (Figure 7). The age at diagnosis for severe PWH decreased as GNI increased, from 44 months in low-income countries, to 10 months in high income countries. In mild & moderate PWH, the highest age at diagnosis was in upper-middle countries at 70 months (Figure 8).

There were 429 PWH newly diagnosed in 2023, 149 of which were severe PWH.

Nineteen percent of mild & moderate PWH, and 37% of severe PWH, were diagnosed before 12 months. Forty-eight percent of mild and moderate PWH and 67% of all severe PWH were diagnosed before the age of 5 years (Table 3a, Figure 9).

62
MONTHS
MEDIAN AGE AT DIAGNOSIS
FOR MILD AND MODERATE PWH

20
MONTHS
MEDIAN AGE AT DIAGNOSIS
FOR SEVERE PWH

Figure 7

Age at diagnosis by region

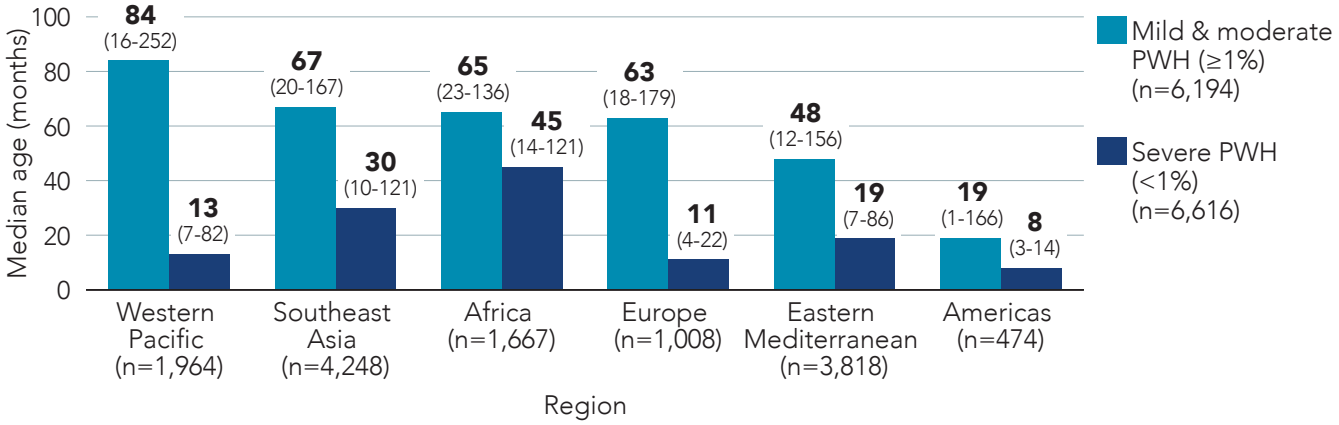


Figure 8

Age at diagnosis by gross national income

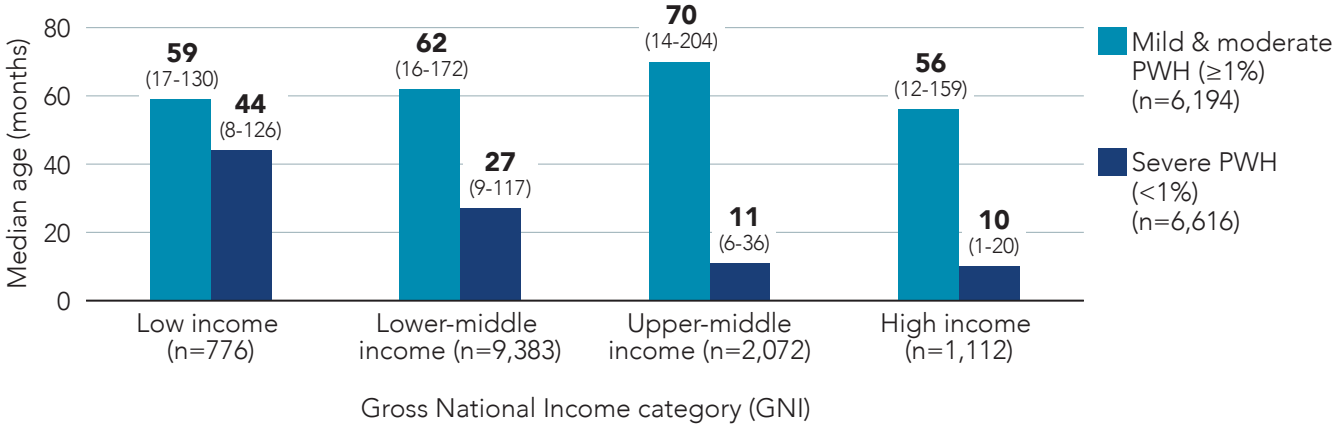
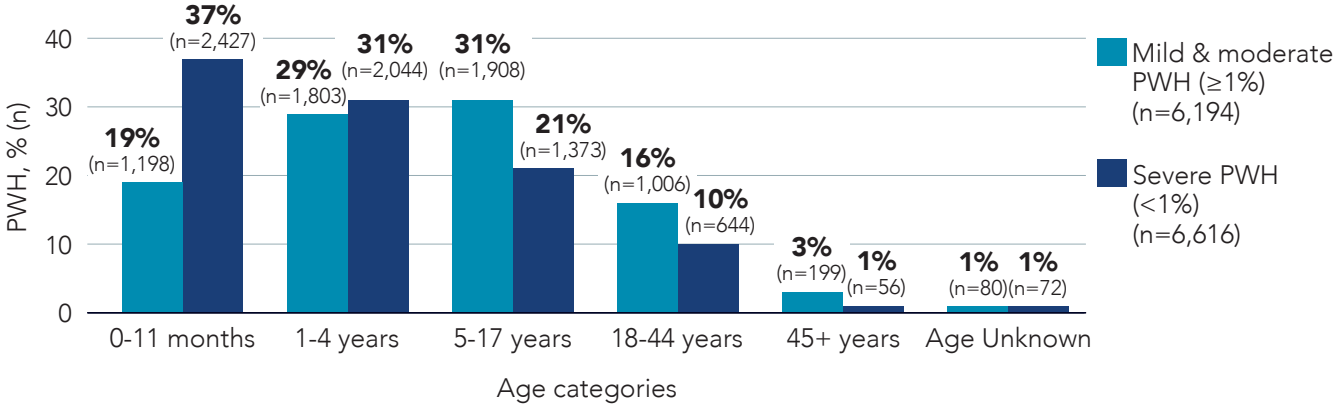


Figure 9

Age distribution of PWH at diagnosis



AGE AT FIRST BLEED AND FIRST JOINT BLEED

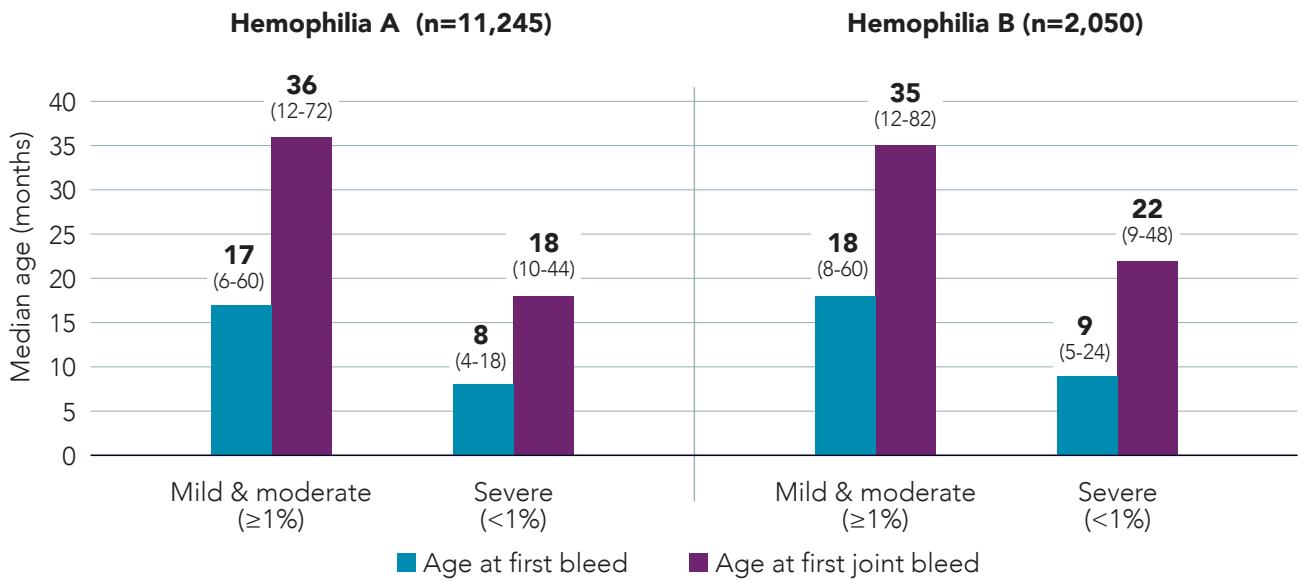
The median age at first bleed and first joint bleed were 11 and 24 months, respectively, for all PWH.

For people with severe hemophilia A, the median age at first bleed was 8 months and the median age at first joint bleed was 18 months (Figure 10).

For people with severe hemophilia B, the median age at first bleed was 9 months and the median age at first joint bleed was 22 months (Figure 10).

Figure 10

Age at first bleed and first joint bleed by severity, Hemophilia A & B, months, median (IQR)



EMPLOYMENT

Of the 2,634 adult (≥18 years old) severe PWH that had their employment status reported, 36% were employed either part-time or full-time. Hemophilia affected the employment status of 20% of PWH, forcing them into part-time employment, long-term sick leave, unemployment or retirement (Table 4).

TABLE 4
Employment

	Mild & moderate PWH* (n=2,463)	Severe PWH* (n=2,634)
Employment status reported		
Employed full-time or part-time	980 (40%)	952 (36%)
Employed part-time due to hemophilia	187 (8%)	232 (9%)
Long term sick leave due to hemophilia	23 (1%)	24 (1%)
Not employed due to hemophilia	131 (5%)	231 (9%)
Retired due to hemophilia	24 (1%)	19 (1%)
Student	804 (33%)	818 (31%)
Other	314 (13%)	358 (14%)

* 126 PWH with unknown severity were excluded.

20% OF ADULTS WITH SEVERE HEMOPHILIA REPORT THEIR EMPLOYMENT STATUS IS NEGATIVELY AFFECTED BY HEMOPHILIA

HEMOPHILIA CLINICAL DATA

THE HEMOPHILIA CLINICAL DATA REPRESENT CLINICAL EVENTS WHICH OCCURRED IN 2023.

TABLE 5

Bleeding events summary, 2023

	Mild & moderate PWH* (n=5,721)	Severe PWH* (n=6,333)
Bleeds per patient, mean (SD)	4.4 (7.0)	7.3 (11.5)
Patients with 0 bleeds in 2023, n (%)	436 (8%)	624 (10%)
Target joints, n (%)**		
≥1	431 (8%)	1,011 (16%)
Total bleeding events$, n	7,943	20,295
Location of bleed, n (%)		
Joint	5,877 (74%)	16,434 (81%)
Muscle	1,087 (14%)	2,174 (11%)
Gastrointestinal	84 (1%)	84 (<1%)
Heavy menstrual bleeding	37 (<1%)	8 (<1%)
Central nervous system	25 (<1%)	27 (<1%)
Neck/Throat	41 (1%)	11(<1%)
Other location	833 (10%)	1,611 (8%)

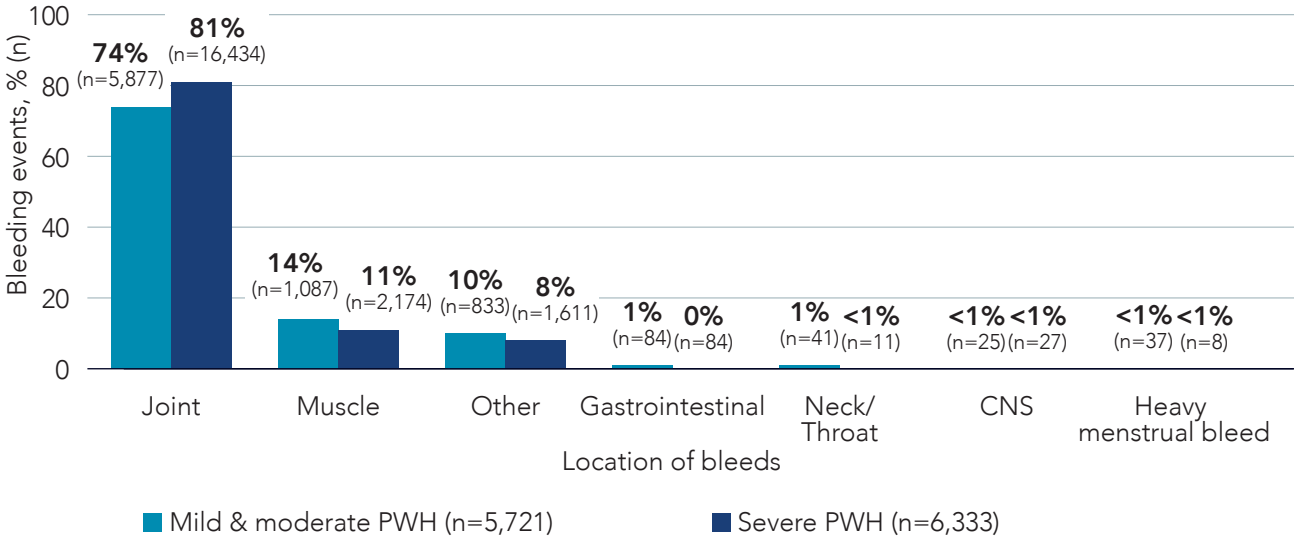
* 532 PWH with unknown severity were excluded. 2023 Data for 757 PWH from Czechia were not available at the time of publication.
** Includes PWH who reported at least one target joint in 2023; Target joints are defined as '3 or more spontaneous bleeds into a single joint within a consecutive 6 month period. Where there have been ≤2 bleeds into the joint within a consecutive 12-month period the joint is no longer considered a target joint'[4].
$ It is possible that PWH had bleeds in more than one location.

BLEEDING EVENTS

In 2023, a total of 29,071 bleeds were reported by PWH. Of these, 22,937 (79%) were joint bleeds, 3,427 (12%) were muscle bleeds and 54 (<1%) were central nervous system (CNS) bleeds. A total of 20,295 bleeds were reported for people with severe hemophilia. The distribution of bleeding events in people with severe hemophilia by location was similar to that of mild & moderate PWH (Figure 11).

Figure 11

Location of bleeding events, % (n)



ANNUALIZED BLEEDING RATE AND ANNUALIZED JOINT BLEEDING RATE

The annualized bleeding rate (ABR) and annualized joint bleeding rate (AJBR) were calculated by annualizing the number of bleeds, and number of joint bleeds respectively. ABR and AJBR were calculated based on the total number of bleeds reported at visits in 2023, divided by the observation period in days, and annualized. The calculation used is: (Number of bleeds / observation period in days) x 365.25. If the interval between the first visit of 2023 and the previous visit is higher than 18 months, the reported bleed of this interval is excluded from the analysis. Only total observation periods of greater than 30 days were used. In the event that a patient did not have a visit in 2023 or an observation period less than 30 days, the ABR and AJBR were not calculated. In figures 14 and 15, patients with 0 bleeds in 2023 were excluded. It is assumed that patients with 0 bleeds in a year are receiving the treatment necessary to prevent bleeding. This allows for a more in-depth analysis of the need for care when observing ABR and AJBR by economic category or region.

ABR

The median (IQR) ABR was 4 (1-10) for all PWH, varying by GNI (Figure 12) and region (Figure 14). High income and upper-middle income countries have an ABR of 0 (Figure 12). Figures 16 and 18 shows ABR with PWH with 0 bleeds excluded by GNI and Region; the ABR for all PWH is 6 (3-13) and decreases as income increases and varies across regions.

AJBR

The median (IQR) AJBR was 2 (0-8) for all PWH, varying by GNI (Figure 13) and region (Figure 15). The AJBR observed in low-income countries was 2 (0-8) and 0 for high income countries. Figure 17 and 19 shows AJBR with PWH with 0 bleeds excluded by GNI and Region; the AJBR was 6 (2-12) and it decreased from 8 (4-15) to 2 (2-4) when comparing low income to high income countries, and varies across regions.

Figure 12

Median ABR for all PWH by GNI

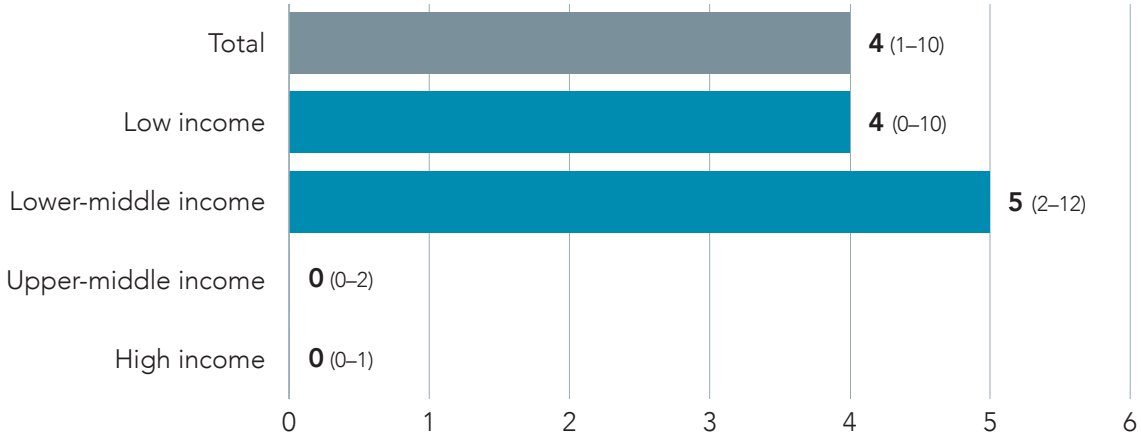


Figure 13

Median AJBR for all PWH by GNI

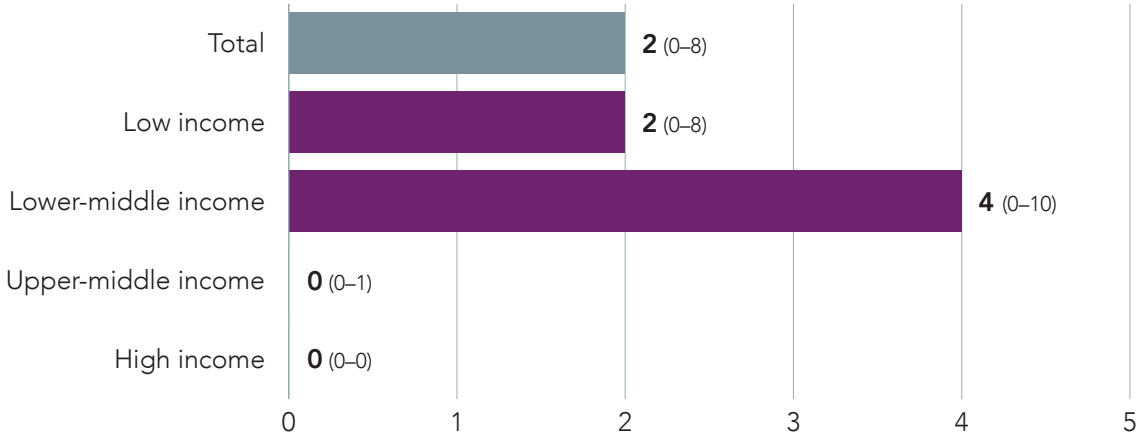


Figure 14

Median ABR for all PWH by region

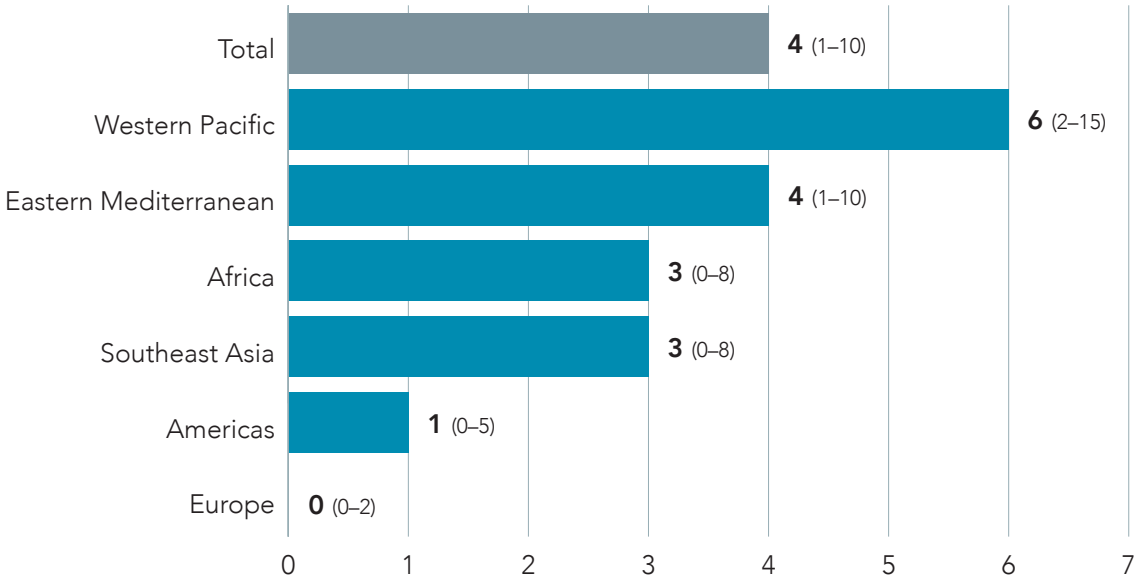


Figure 15

Median AJBR for all PWH by region

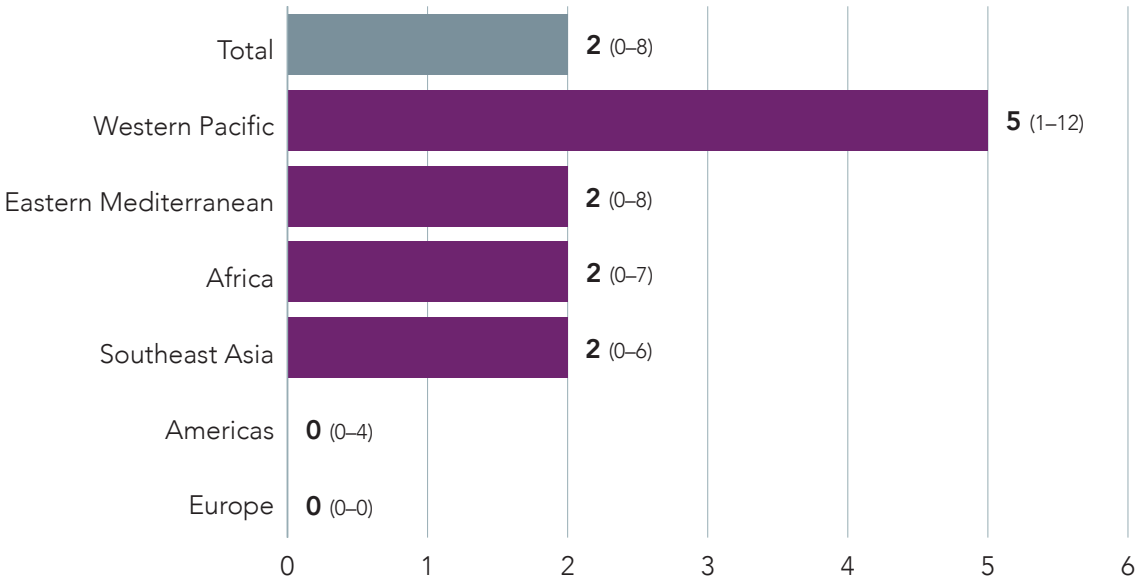


Figure 16

Median ABR for all PWH by GNI (patients with 0 bleeds excluded)

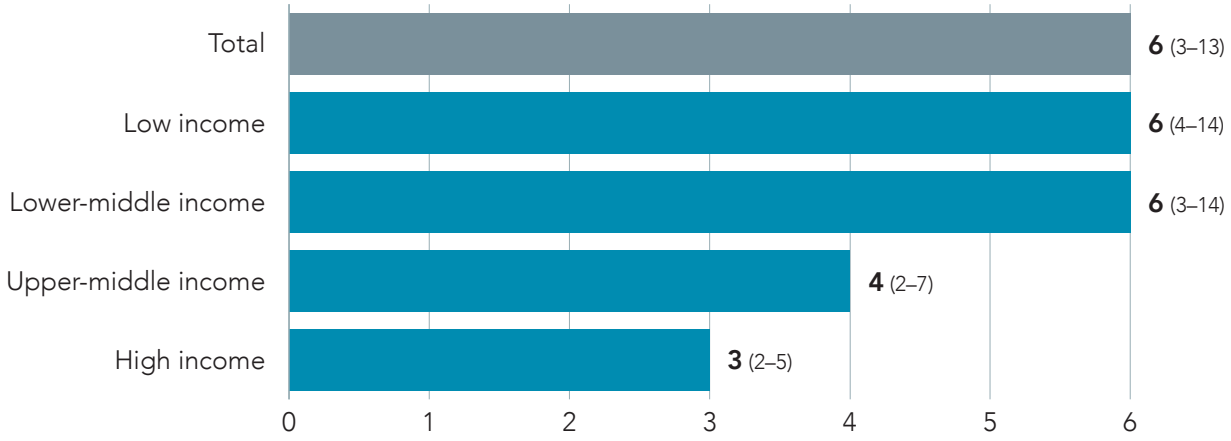


Figure 17

Median AJBR for all PWH by GNI (patients with 0 bleeds excluded)

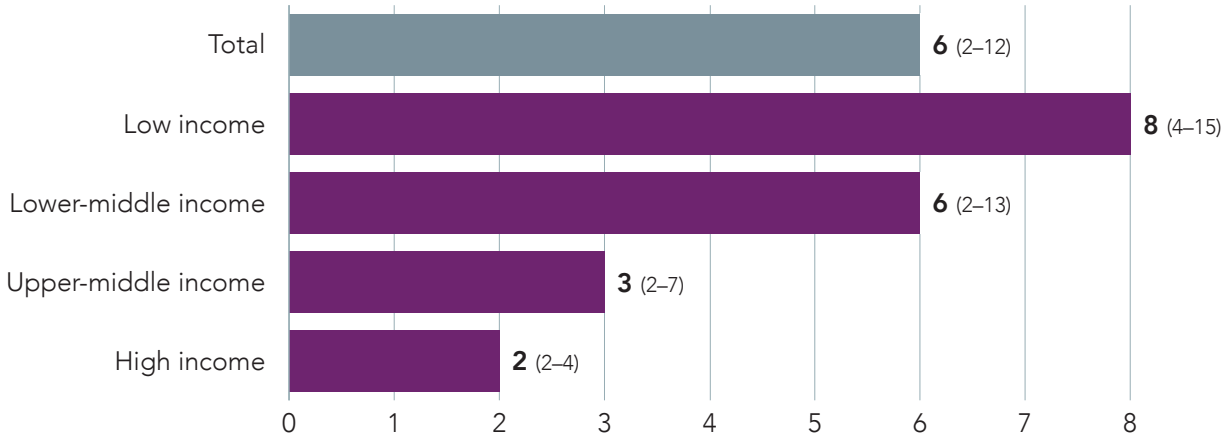


Figure 18

Median ABR for all PWH by region (patients with 0 bleeds excluded)

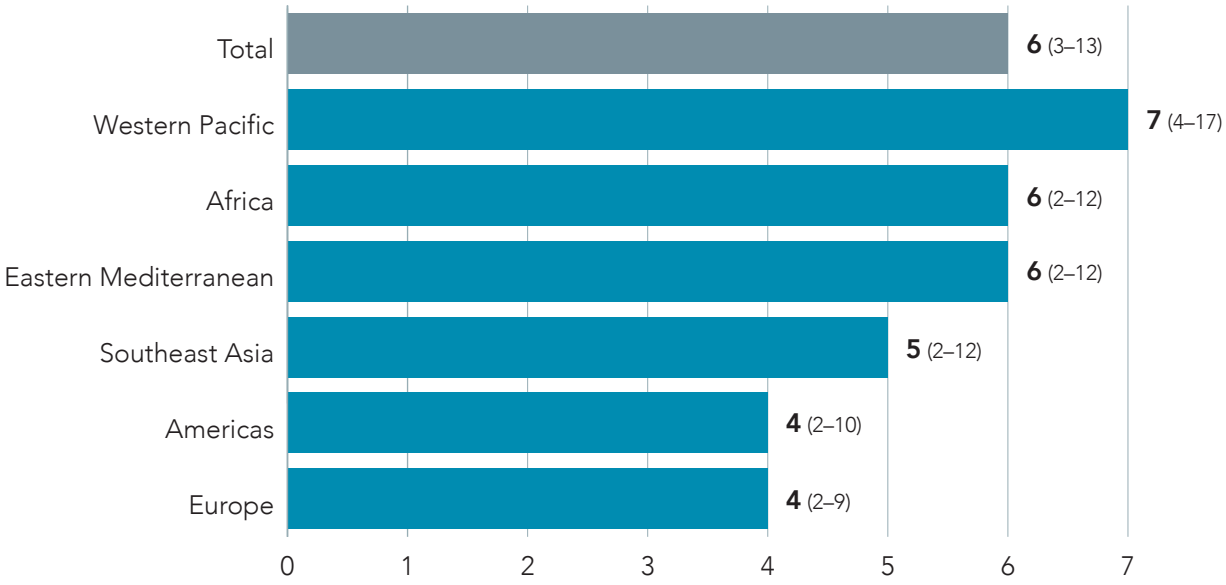
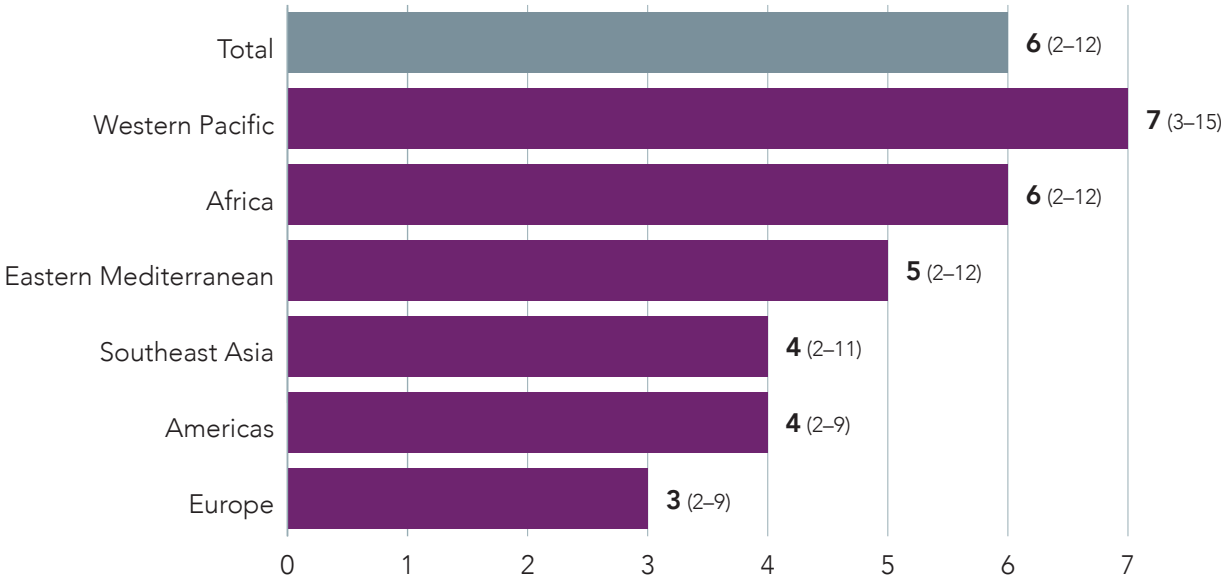


Figure 19

Median AJBR for all PWH by region (patients with 0 bleeds excluded)



ABR AND AJBR WITH AND WITHOUT PROPHYLAXIS

The median ABR for mild & moderate PWH was 4 for those with and without prophylaxis treatment in 2023. For severe PWH, the median ABR decreased from 5 to 2 for those with prophylaxis treatment in 2023 (Figure 20). Figure 21 shows the AJBR for PWH with and without prophylaxis, which illustrates the same pattern as ABR. AJBR for severe PWH decreases from 4 to 1 with prophylaxis treatment.

Figure 20

Median ABR for all PWH - With and without prophylaxis treatment

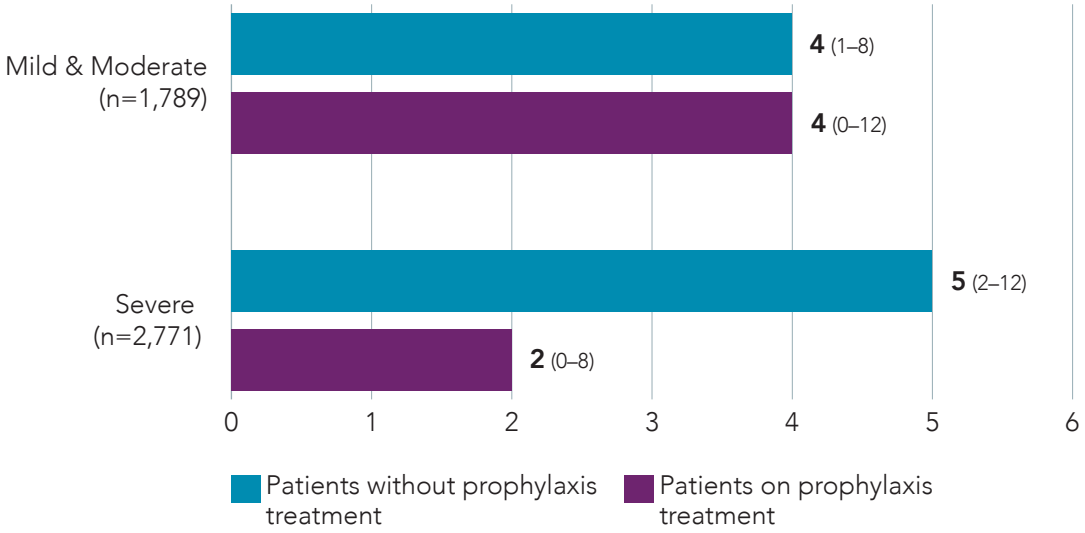
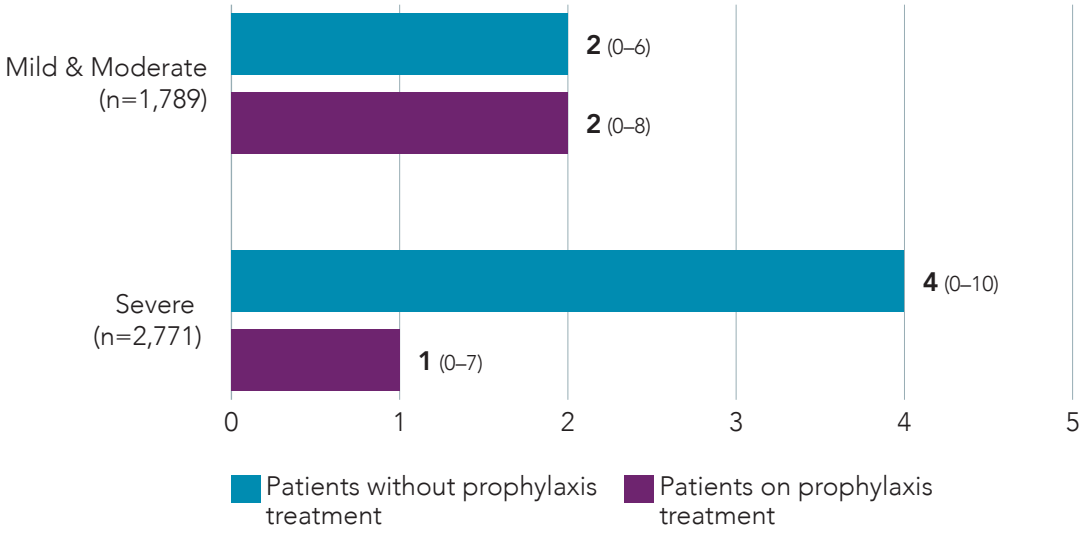


Figure 21

Median AJBR for all PWH - With and without prophylaxis treatment



TARGET JOINTS

Eight percent of mild & moderate PWH, and 16% of severe PWH who reported having at least 1 target joint in 2023. The percent of PWH reporting a target joint varied by region, ranging from 26% to 2% in severe PWH (Figure 22).

Figure 22

PWH with at least 1 target joint by region

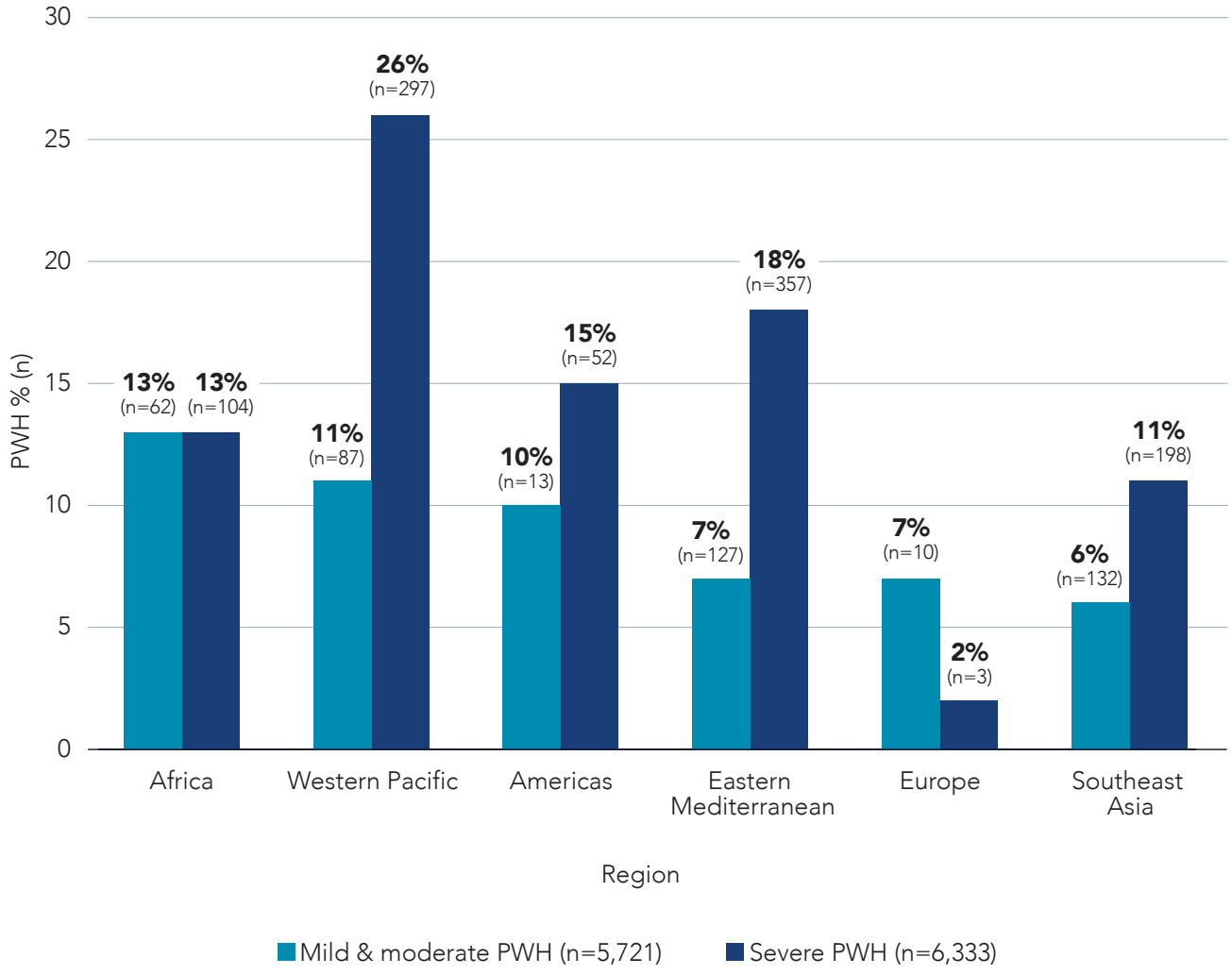


TABLE 6
Inhibitor summary, 2023

	Mild & moderate PWH (n=5,721)*	Severe PWH (n=6,333)*
Patients with a history of an inhibitor**, n (%)	191 (3%)	686 (11%)
Inhibitor testing in 2023		
Tested†, n (%)	111 (2%)	382 (6%)
Positive test results	14 (13%)	81 (21%)
Negative test results	97 (87%)	301 (79%)
Newly diagnosed with an inhibitor††, n (%)	8 (<1%)	24 (<1%)
Patients with suspected inhibitor, but no testing available$, n (%)	1 (<1%)	2 (<1%)

* 2023 Data for 757 PWH from Czechia were not available at the time of publication.
** Unique number of patients who had an inhibitor prior to registration in the WBDR or a positive test prior to 2023.
† Unique number of patients who had an inhibitor test in 2023. Testing methods include Bethesda, Nijmegen-Bethesda, and mixing study (aPTT).
†† Unique number of patients who never had an inhibitor in the past, were tested in 2023, and had a positive result.
$ Includes all PWH with a baseline visit in 2023.

Data on inhibitor testing is collected at baseline visit (for 6 months prior) and at each follow-up visit thereafter. In this report, the number of PWH with a positive inhibitor test is defined as any PWH who has had at least 1 positive inhibitor test in 2023. In 2023, 111 mild & moderate and 382 severe PWH were tested for inhibitors. Fourteen (13%) and 81 (21%) of PWH tested positive for an inhibitor for mild & moderate and severe PWH respectively. There were a total of 33 (<1%) PWH newly diagnosed with an inhibitor (no history of inhibitors and no prior positive test reported), with <1% being severe PWH (Table 6 and Figure 23).

Figure 23
Severe PWH with inhibitor test, 2023 (n=382)

TABLE 7a

Hemophilia related hospitalizations summary, 2023

	Mild & moderate PWH (n=5,721)*	Severe PWH (n=6,333)*
Patients hospitalized**, n (%)	358 (6%)	659 (10%)
Total hospitalizations†, n	1,108	3,189
Days per hospitalization, median (IQR)	8 (4-18)	14 (6-30)
Number of hospitalizations per patient$, median (IQR)	1 (1-4)	3 (1-7)

TABLE 7b

	Mild & moderate PWH (n=5,721)*	Severe PWH (n=6,333)*
Reason for hospitalizations, n (%)		
Joint bleeding	712 (12%)	2,391 (38%)
Other muscle bleeding	162 (3%)	417 (7%)
Other bleeding	110 (2%)	234 (4%)
Soft tissue bleeding	25 (<1%)	45 (<1%)
Gastrointestinal bleeding	17 (<1%)	25 (<1%)
Surgery	15 (<1%)	22 (<1%)
Intracranial bleeding	13 (<1%)	19 (<1%)
Epistaxis	5 (<1%)	1 (<1%)
Psoas muscle bleeding	4 (<1%)	9 (<1%)
Neck hematomas	1 (<1%)	1 (<1%)
Other	76 (1%)	186 (3%)

* 532 PWH with unknown severity were excluded. 2023 Data for 757 PWH from Czechia were not available at the time of publication.
** Number of unique PWH hospitalized.
$ Based only on patients who were hospitalized.
† Hospitalization is defined as having at least 1 overnight stay in the hospital.

In 2023, 358 (6%) mild & moderate PWH and 659 (10%) severe PWH experienced a total of 4,329 hemophilia related hospitalizations. The median (IQR) length of hospital stay for mild & moderate PWH and severe PWH was 8 days of 14 days respectively (Table 9a). The most common reason for hospitalization was joint bleed for both hemophilia A and B patients (70% and 61% respectively) (Figures 24 and 25). In total, 34 hospitalizations were for an intracranial bleed; 24 (1%) were among hemophilia A patients and 10 (<1%) were among hemophilia B patients. PWH with hemophilia type unknown, who were hospitalized are not included in the graphs below (Figures 24 and 25).

Figure 24

Reason for hospitalization in hemophilia A patients (n=3,693)

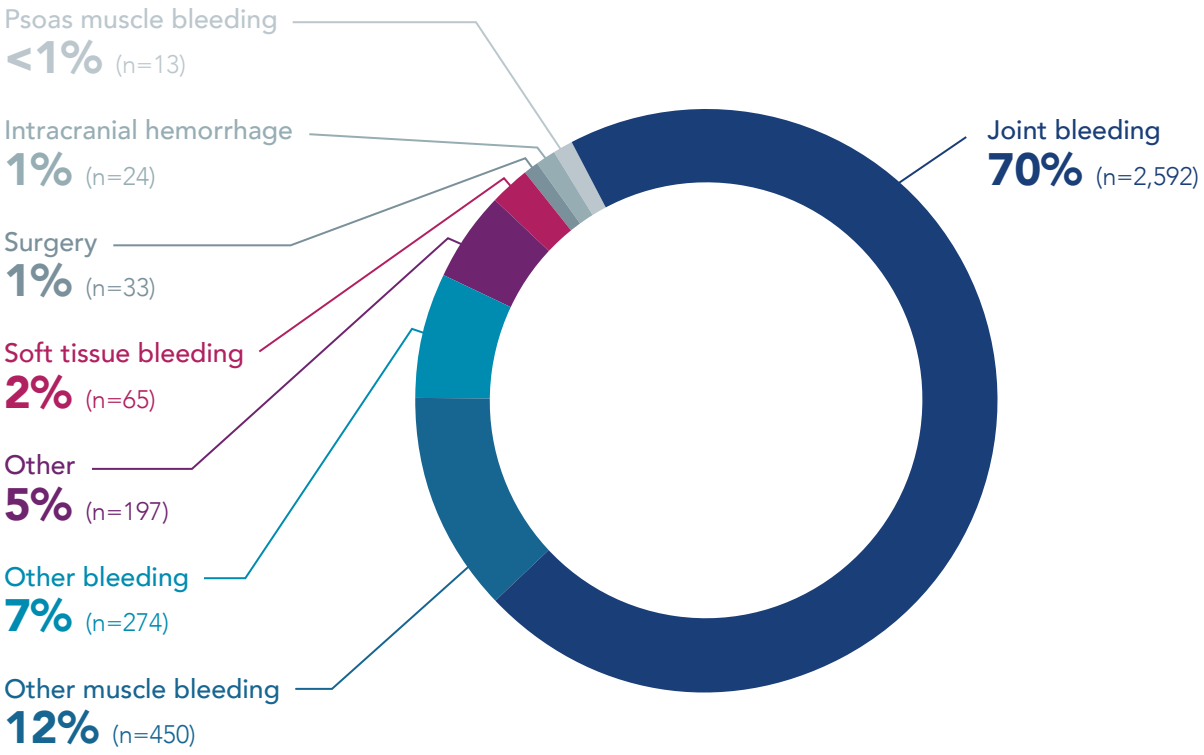
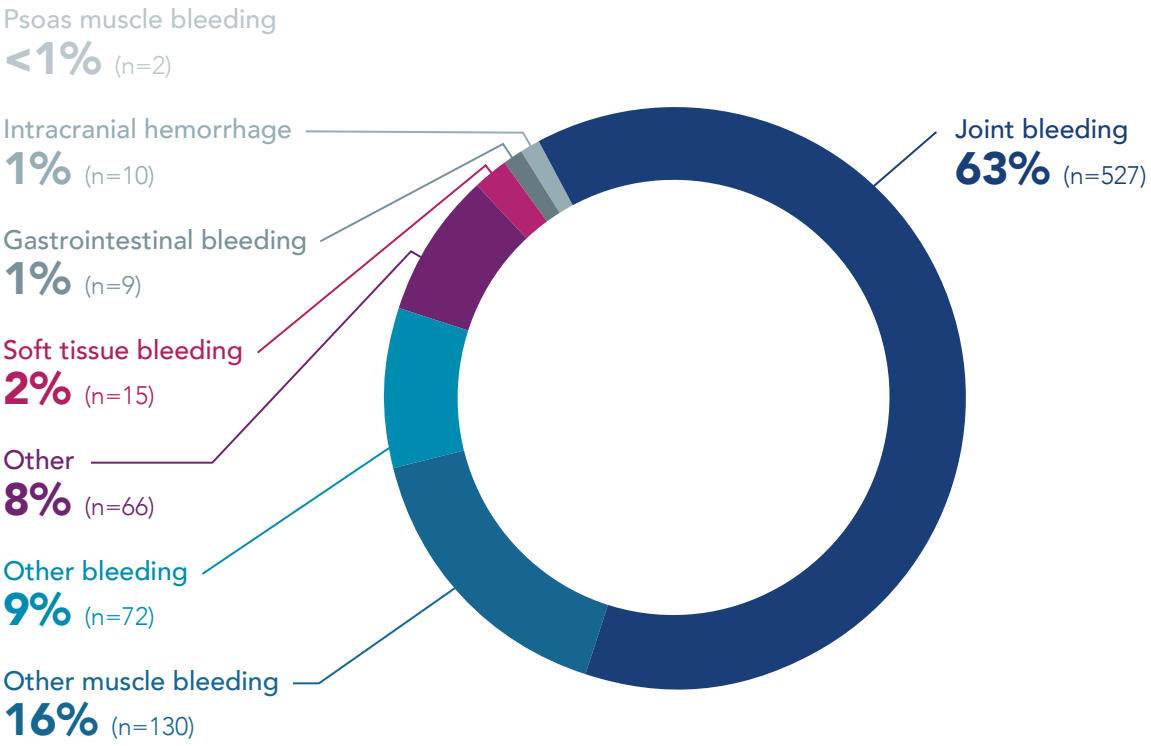


Figure 25

Reason for hospitalization in hemophilia B patients (n=835)



TREATMENT

TABLE 8

Treatment summary, 2023*

	Mild & Moderate PWH (n=5,721)*	Severe PWH (n=6,333)*
Received at least 1 prophylaxis treatment in 2023, n (%)**	446 (8%)	1,775 (28%)
Hemophilia A, n (%)	360 (8%)	1,574 (29%)
FVIII, standard half-life	200 (56%)	979 (62%)
FVIII, extended half-life	62 (17%)	207 (13%)
Bypassing agent	1 (<1%)	9 (<1%)
Non-factor product	128 (36%)	487 (31%)
Other	10 (3%)	47 (3%)
Hemophilia B, n (%)	86 (9%)	201 (23%)
FIX, standard half-life	36 (42%)	104 (52%)
FIX, extended half-life	39 (45%)	80 (40%)
Bypassing agent	0 (0%)	3 (2%)
Non-factor product	0 (0%)	5 (3%)
Other	20 (23%)	31 (15%)

* 2023 Data for 757 PWH from Czechia were not available at the time of publication. 48 people with hemophilia type unknown and 532 PWH with unknown severity were excluded from this analysis.
** Number of unique PWH who received prophylaxis treatment in 2023. This includes patients who started prophylaxis treatment or had an on-going treatment in 2023. Patients may be receiving treatments that fall under multiple categories.

PROPHYLAXIS USE

A total of 2,278 (18%) PWH received prophylaxis as treatment in 2023. Twenty-eight percent of severe PWH received prophylaxis in 2023; 89% of these were PWH A and 11% were PWH B (Table 8).

Figure 26

Percentage of PWH receiving prophylaxis in 2023 by region*

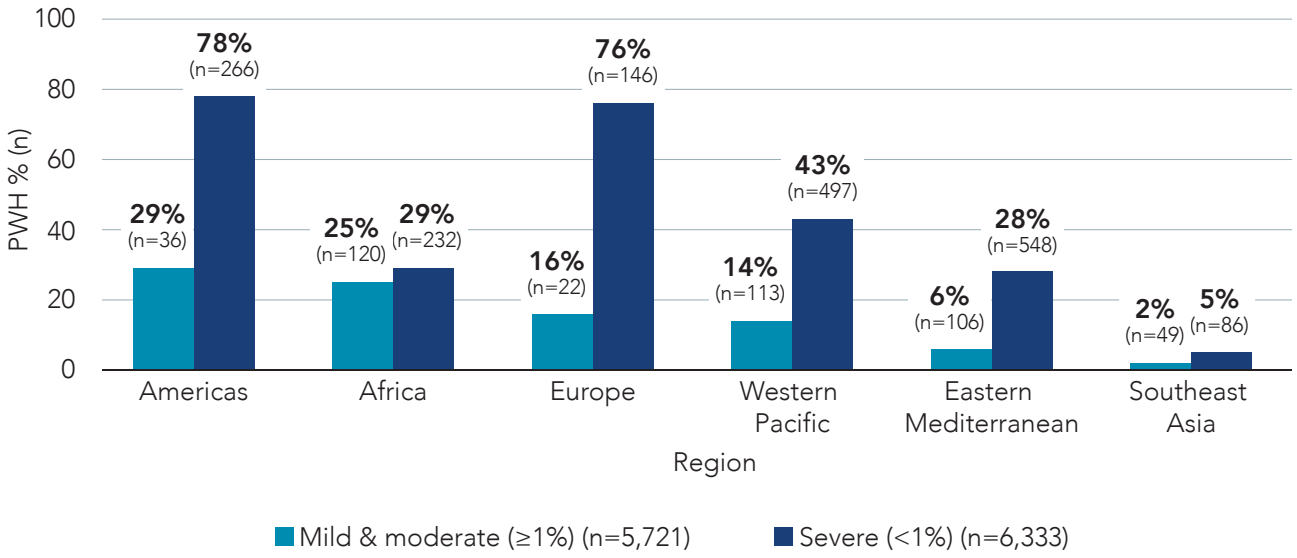
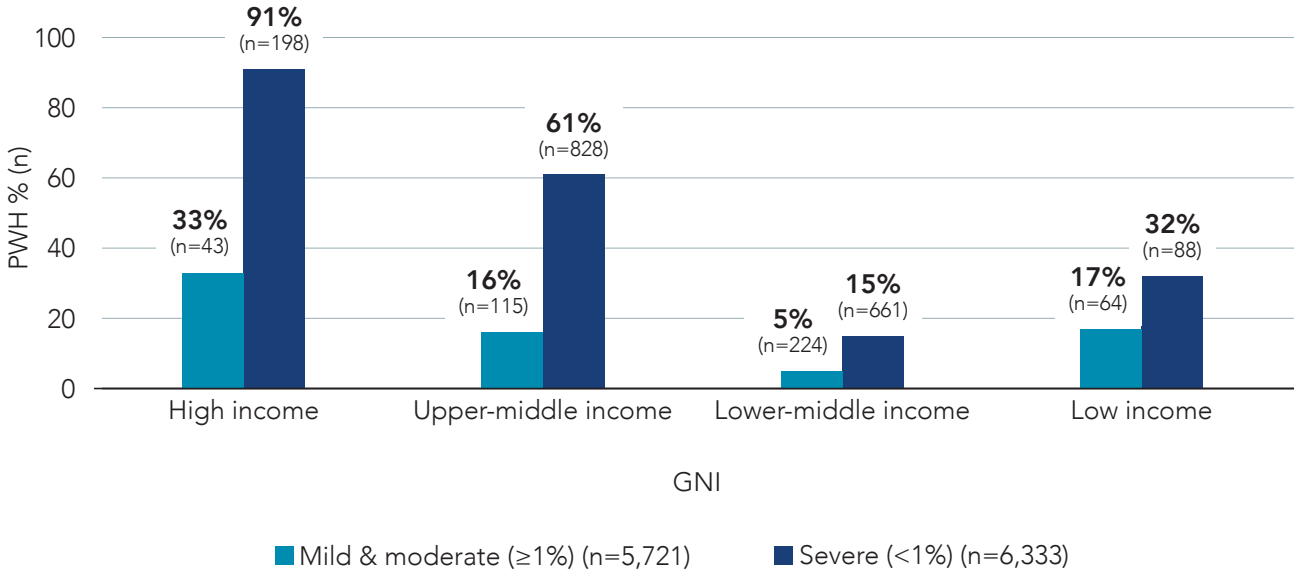


Figure 27

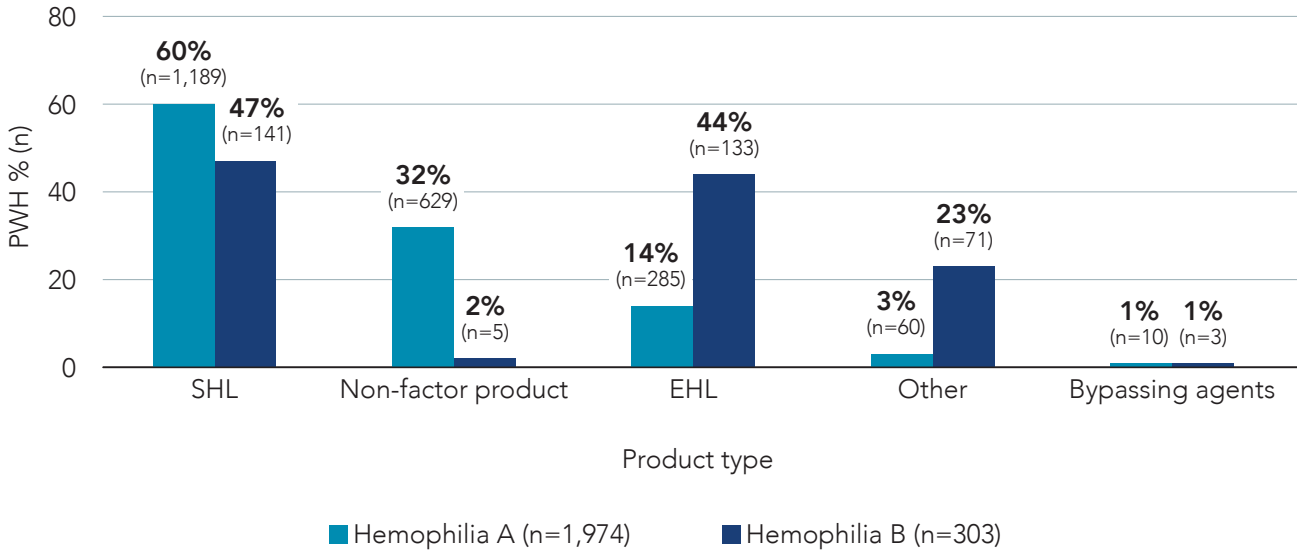
Percentage of PWH receiving prophylaxis in 2023 by GNI



For PWH treated with prophylaxis, standard half-life (SHL) clotting factor concentrates were the most common type of treatment used in 2023 (63% of PWH A on prophylaxis, and 52% of PWH B on prophylaxis). Twenty eight percent of people with hemophilia A received non-factor product treatments (Table 8, Figure 28).

Figure 28

Distribution of product type among PWH on prophylaxis treatment



DATA INCLUDED IN THE WBDR 2023 DATA REPORT **VON WILLEBRAND DISEASE**

45
HTCs ENROLLED

22
COUNTRIES REPRESENTED

999
ENROLLED PATIENTS

TABLE 9

VWD Participation Summary

Countries, n	22
Hemophilia treatment centres*, n	45
People with VWD, n	999
Distribution of PwVWD by region†, n (%)	
Africa	22 (2%)
Americas	2 (<1%)
Eastern Mediterranean	649 (65%)
Europe	274 (27%)
Southeast Asia	15 (2%)
Western Pacific	37 (4%)
Distribution of PwVWD by GNI§, n (%)	
High income	274 (27%)
Upper-middle income	41 (4%)
Lower-middle income	674 (68%)
Low income	10 (1%)

* HTCs included are those with Institutional Board Review approval and have enrolled at least 1 PwVWD by December 31, 2023.
† Regions based on WHO regional groupings[2].
§ GNI = Gross National Income; Gross National Income categories based on The World Bank Group 2023 rankings for "Gross national income (GNI) per capita, Atlas method (current US$)"[3].

DISTRIBUTION OF PwVWD

The regional classification used in the WBDR is based on the WHO regional classification[1]. The majority of PwVWD are from the Eastern Mediterranean region (Iran, Iraq, Morocco, Pakistan, Sudan, Syria and Tunisia) and Europe (Czechia, Portugal), representing 65% and 27% of PwVWD respectively (Figure 29).

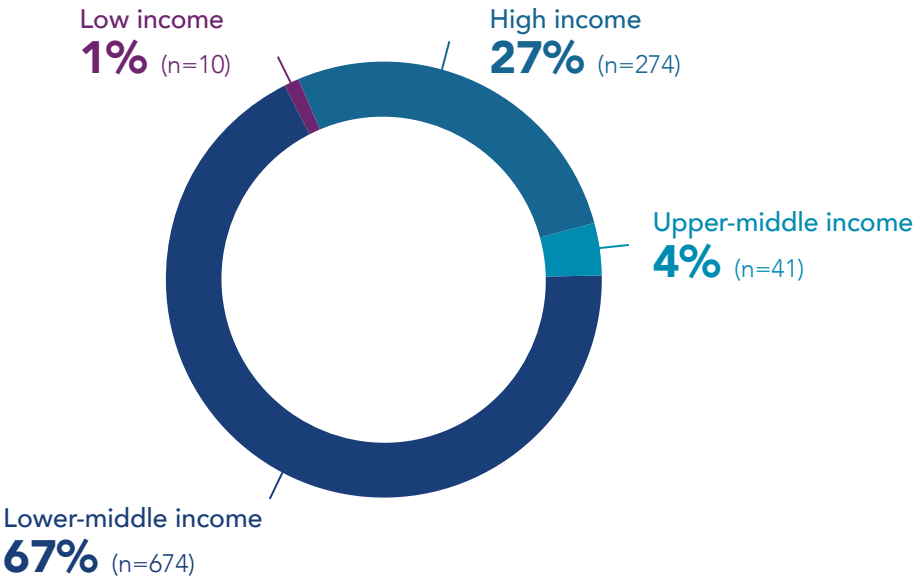
Figure 29

Distribution of PwVWD by region

The distribution of participants by Gross National Income (GNI) per capita[2], demonstrates that the majority of the PwVWD are from lower-middle income countries (67%), followed by high, upper-middle, and low income representing 27%, 4% and 1% respectively (Figure 30).

Figure 30

Distribution of PwVWD by GNI

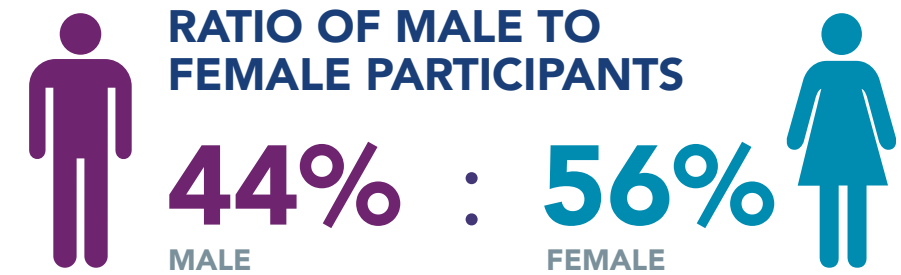


VWD DEMOGRAPHICS

TABLE 10
VWD Demographics summary

	VWD (n=999)
Sex, n (%)	
Male	439 (44%)
Female	560 (56%)
Age of PwVWD[$]	
Age, years, median (IQR)	21 (12-36)
Pediatrics (<18 years)	432 (43%)
Adults (18 years)	567 (57%)
VWD Type, n (%)	
Type 1	224 (22%)
Type 2	189 (19%)
Type 3	547 (55%)
Platelet-Type VWD	1 (<1%)
Unknown	38 (4%)

IQR=interquartile range
[$] Age was calculated as of December 31, 2023.

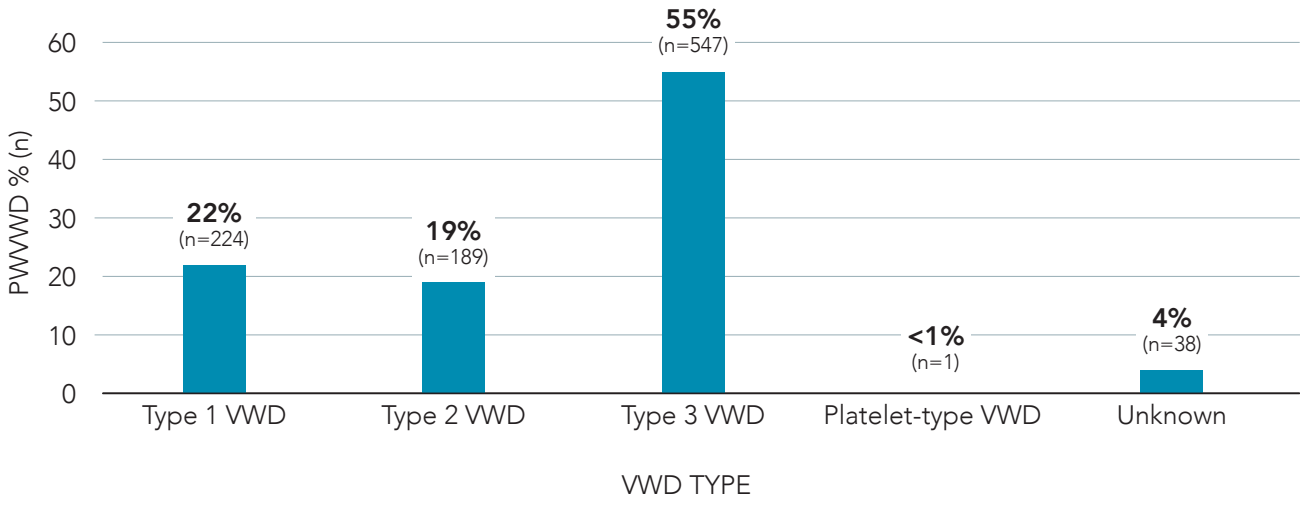


VWD TYPE

Overall, 56% (n=560) of participants were female and 44% (n=439) were male (Table 10). Fifty-five percent (n=547) of patients had Type 3 VWD, while 22% and 19% had Type 1 and Type 2 respectively (Table 10, Figure 31).

Figure 31

VWD Type



VWD CLINICAL DATA

THE VWD CLINICAL DATA REPRESENT CLINICAL EVENTS WHICH OCCURRED IN 2023.

TABLE 11

Bleeding events summary, 2023*

Age at first bleed, months, median (IQR)	4 (2-24)
Patients with 0 bleeds in 2023, n (%)	154 (33%)
Total bleeding events, n**	2,426
Location of bleed, n (%)	
Joint	297 (12%)
Muscle	125 (5%)
Heavy menstrual bleeding	468 (32%)
Gastrointestinal	53 (2%)
Neck/Throat	21 (1%)
Other bleeds	1,408 (58%)

* 2023 Data for PwVWD from Czechia were not available at the time of publication.
** It is possible that PwVWD had bleeds in more than one location.

4 MONTHS
MEDIAN AGE AGE AT FIRST BLEED FOR PEOPLE WITH VWD

24%
PEOPLE WITH VWD WITH AT LEAST 1 TREATMENT IN 2023

2%
PEOPLE WITH VWD ON PROPHYLAXIS IN 2023

WBDR DATA QUALITY ACCREDITATION PROGRAM

The primary aim of the WBDR Data Quality Accreditation (DQA) Program is to standardize data collection procedures across HTCs and ensure high quality data are entered in the WBDR. A robust data cleaning and validation process is systematically applied to enhance data completeness, accuracy, and consistency.

Comprehensive assessment of data is conducted along two pivotal dimensions:

- Completeness: Requires all data fields to be fully complete.
- Accuracy: Enforces the validity and consistency of all data fields.

The WBDR data quality team collaborates closely with HTCs, offering comprehensive training and constructive feedback to uphold high data quality standards. Instances of incomplete and inaccurate data are communicated to HTCs through Data Clarification Forms twice a year, accompanied by requests for data updates. At the end of the year, each HTC undergoes an evaluation based on the overall level of data quality at their respective sites, adhering to the WBDR Data Quality Rating classification levels (refer to Figure 32).

73% (71) OF HTCs

ACHIEVED THE HIGHEST LEVEL OF DATA QUALITY RATING, AND WERE **CLASSIFIED AS 'EXPERTS'.**
(DATA QUALITY SCORE ≥95%)

In the year 2023, the WBDR team engaged directly with 97 HTCs. Impressively, 71 of these HTCs (73%) were considered 'Experts' in data entry with a score of 95% and above, representing the highest echelon of data quality.

Figure 32

WBDR Data Quality Rating Scale

Note: Data imported through the International Data Integration Program are not verified under the WBDR's Data Quality Accreditation Program.

The WBDR DQA Program allows the WFH and its partnering HTCs to regularly monitor and evaluate data quality in the WBDR, to prevent and manage data quality challenges, and to prioritize areas for improvement. By implementing the WBDR DQA Program, the WFH aims to improve the WBDR's effectiveness, efficiency, and sustainability.

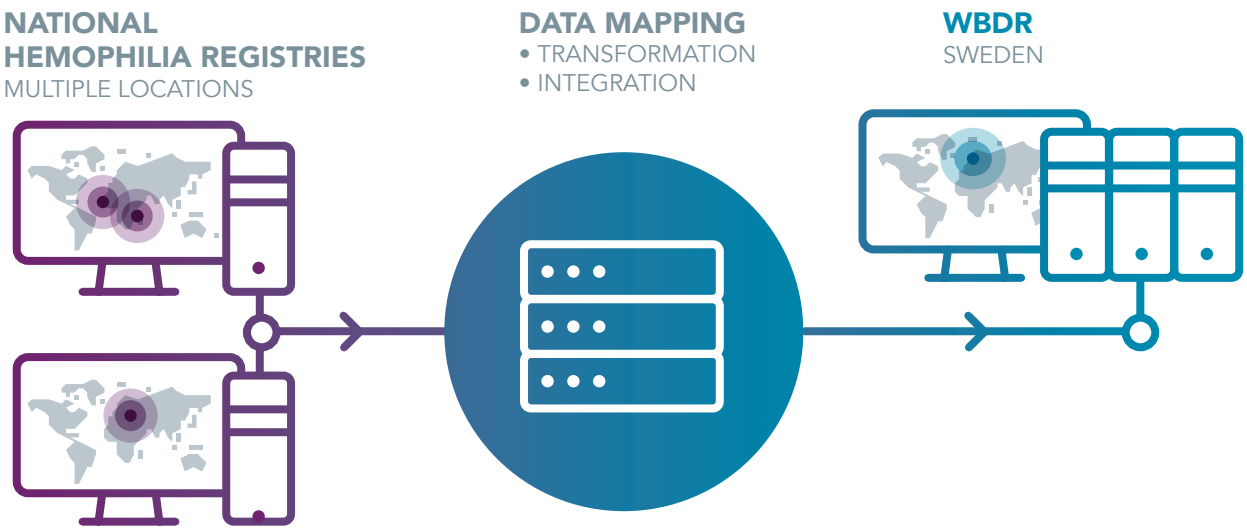
INTERNATIONAL DATA INTEGRATION PROGRAM

Registries, with international collaboration between countries, offer an opportunity to pool sufficient data to increase the knowledge and evidence in rare disorders across different regions and economies. In an effort to combine resources from existing hemophilia registries and maximize the utility of data that currently exist, the WBDR established the International Data Integration Program with the aim of facilitating data transfer from existing patient registries to the WBDR. A protocol to import data from established registries into the WBDR has been developed and tested. In 2019, the WBDR started a data linkage collaboration with the Czech National Haemophilia Programme Registry (CNHPR). Since 2019, de-identified data from the CNHPR are annually imported into the WBDR. To date, data on over 850 PWH have been collected and annually updated, with 31 newly enrolled PWH in 2023. With the addition of the VWD module to the WBDR in 2023, demographic, clinical and diagnostic data on over 270 people with VWD from the CNHP were imported for the first time and will be updated annually.

Since 2021, the Hereditary Bleeding Disorders Registry (HBDR) of the Thai Society of Hematology (TSH) starts collaborating with the WBDR to integrate selected data in the global registry. Eight of 10 HTCs at the university hospitals took part in this collaboration. In 2023, the minimum and extended data sets of over 300 people with hemophilia A and B have been transferred to the WBDR with 8 newly enrolled PWH.

At time of the publication of this report, The TSH and the WFH are evaluating the possibility of integrating the minimum data sets of VWD patients into the WBDR through international data integration program.

The International Data Integration Program is available to interested countries who wish to join this global initiative by sharing their national data and having their PWH represented in the WBDR. Interested individuals are encouraged to contact the WFH at wbdr@wfh.org.



WBDR RESEARCH SUPPORT PROGRAM

The WBDR Research Support Program (RSP) is designed to support HTCs to collect, analyze and use their WBDR data by providing small research funding. This program is open to recognized HTCs from any country as long as they are participating in the WBDR.

In 2023, seven HTCs were awarded funding for their research project. Since 2018, we have provided funding to 46 research projects in 24 countries.

Since 2018:

COUNTRIES

24

RESEARCH PROJECTS

46

HTCs PARTICIPATED

39

AMOUNT AWARDED

>200,000

USD

PUBLICATIONS AT INTERNATIONAL CONFERENCES

(ABSTRACTS AND FEATURED ARTICLES)

23

WBDR HTC FUNDING PROGRAM

The WBDR HTC Funding Program (HFP) is designed to provide funds to support data collection activities at participating WBDR HTCs in low and lower-middle income countries.

The HFP aims to help HTCs improve patient enrolment, the recording of follow-up visits, functional scales, and quality of life measures. Eligible HTCs are compensated based on the number of active patients enrolled in the WBDR or the number of identified hemophilia and von Willebrand patients being followed at the HTC at the time of the application. The funds are allocated for a period of one year.

The impact of the HFP at recipient HTCs is demonstrated by the significant improvement in patient enrolment and overall data collection. HFP recipient HTCs have enrolled 519 new PWH during 2023.

For more information, please visit our webpage.

RECIPIENT COUNTRIES IN 2023

12

AMOUNT AWARDED IN 2023

$26,000 USD

RECIPIENT HTCS IN 2023

20

myWBDR MOBILE APPLICATION

myWBDR is a mobile application designed for WBDR patients diagnosed with hemophilia or VWD. With a primary objective of empowering its users, myWBDR offers an accessible platform to effortlessly track and monitor bleeding episodes, pain levels, treatments, and overall health status using EQ-5D-5L and PROBE questionnaires.

It is available in the following languages: Arabic, English, French, Hindi, Portuguese, Russian, Spanish, Thai and Vietnamese.

myWBDR was officially launched in May 2022. Since then, it has been adopted in 13 countries and has accumulated over 700 bleed records and more than 5,000 treatment records, signifying its impactful role in gathering patient-reported data, contributing to improved clinical management, and the generation of valuable evidence for bleeding disorders research and decision-making.

myWBDR also serves as an important tool for healthcare providers (HCPs) to monitor the health status of their patients and enhance the quality of care and treatment provided. It is accessible through HTCs participating in the WBDR. Those interested can reach out to their HCP to initiate the sign-up process. Further inquiries or requests for additional information about myWBDR can be directed to myWBDR@wfh.org.

APPENDIX 1 – WBDR DATA SETS

WBDR Data Set

Demographics	Diagnostics	Clinical
Date of birth	**Date of diagnosis**	**Bleeding events**
Gender	**Hemophilia type**	**Target joints**
Country of residence	**Hemophilia severity**	**Treatments**
Employment	**Hemophilia factor level**	**Inhibitor status**
Education	**Inhibitor history**	**Hospitalization**
Marital status	**Treatment history**	**Mortality**
	Bleeding history	Adverse events
	Genetic testing	Comorbidities
	Blood type	Functional scales*
	Family history	Quality of life scales**
		COVID-19

Fields identified in bold represent the minimal data set, which are the mandatory data fields.
* Functional scales include: Haemophilia Joint Health Score, Joint Disease, Range of Motion, WFH Gilbert Score, Functional Independence Score for Haemophilia.
** Quality of life scale: EQ-5D-5L.

APPENDIX 2 – PARTICIPATING HTCs

Country	City-Clinic
Algeria	• Annaba - Service d'hématologie CHU Annaba • Constantine - Unité hémophilie et maladies hémorragiques héréditaires
Argentina	• Bahía Blanca - CARDHE • Buenos Aires - Fundación de la Hemofilia and Instituto De Investigaciones Hematológicas "Dr. Mariano R. Castex"
Bangladesh	• Chittagong - Chittagong Medical College Hospital • Dhaka - Bangabandhu Sheikh Mujib Medical University • Dhaka - Dhaka Medical College • Dhaka - Dhaka Shishu Hospital • Dhaka - Lab One Foundation • Rajshahi - Rajshahi Medical College & Hospital
Barbados	• Bridgetown - Queen Elizabeth Hospital
Belgium	• Woluwe-Saint-Lambert - Cliniques Universitaires Saint-Luc
Burkina Faso	• Ouagadougou - CHU Pédiatrique Charles de Gaulles
Cameroon	• Yaoundé - CHU Yaoundé
Cuba	• Havana - Instituto de Hematología e Inmunología
Czechia	• Brno - University Hospital: Dpt. of Clinical Hematology • Brno - University Hospital: Dpt. of Pediatric Hematology • České Budějovice - Dpt. of Clinical Hematology • České Budějovice - Pediatric Dpt. • Hradec Králové - University Hospital: Dpt. of Pediatric Medicine • Liberec - Regional Hospital: Dpt. of Clinical Hematology • Olomouc - University Hospital: Dpt. of Pediatric Medicine • Olomouc - University Hospital: Haemato-Oncology Dpt. • Ostrava - University Hospital: Blood centre • Ostrava - University Hospital: Dpt. of Pediatric Medicine • Pilsen - Hemacentrum • Pilsen - University hospital: Dpt. of Biochemistry and Hematology • Pilsen - University hospital: Pediatric Dpt. • Prague - University Hospital in Motol: Dpt. of Pediatric Hematol-ogy and Oncology • Ústí nad Labem - Masaryk Hospital: Dpt. of Clinical Hematology • Ústí nad Labem - Masaryk Hospital: Pediatric Dpt. - Hematology
Egypt	• Cairo - Pediatric Hemophilia Centre, Ain Shams University • Giza - Shabrawishi Hospital • Mansoura - Mansoura University Children Hospital • Zagazig - pediatrics department, Zagazig University
El Salvador	• San Salvador - Hospital Nacional de Ninos Benjamin Bloom
Ethiopia	• Addis Ababa - Tikur Anbessa Hospital
Ghana	• Kumasi - Komfo Anokye Teaching Hospital
Guinea	• Conakry - Hopital National Ignace Deen, CHU de Conakry

Country	City-Clinic
India	• Aluva - Haemophilia Treatment Centre, District Hospital • Bhopal - Gandhi Medical College • Dibrugarh - Assam Medical College Hemophilia Treatment Center • Kochi - Amrita Institute of Medical Sciences • Ludhiana - Christian Medical College • Manipal - Manipal Academy of Higher Education (MAHE) and Hemophilia Society Manipal Chapter • Noida - Post Graduate Institute of Child Health • Tiruvalla - Believers Church Medical College Hospital
Indonesia	• Banjarmasin - Ulin General Hospital
Iran	• Ahvaz - Baghaei 2 hospital
Iraq	• Baghdad - Hemophilia Center - Medical City • Baghdad - National Center of Hematology - Al-Mustansirya University • Basra - Basra Center for heriditery Blood Diseases
Ivory Coast	• Abidjan - CHU de Yopougon
Kenya	• Eldoret - Moi Teaching and Referral Hospital • Nairobi - Kenyatta National Hospital
Kyrgyzstan	• Bishkek - National Center for Maternity and Childhood • Bishkek - National Center of Oncology and Hematology • Osh - Adult Hematology - Osh Interregional Joint Clinical Hos-pital • Osh - Dept of Pediatric Hematology - Interregional Children's Clinical Hospital
Madagascar	• Antananarivo - CHU Joseph Ravoahangy Andrianavalona (HJRA)
Malawi	• Lilongwe - Kamuzu Central Hospital
Malaysia	• Alor Setar - Hospital Sultanah Bahiyah • Ampang - Hospital Ampang • George Town - Hospital Pulau Pinang • Ipoh - Hospital Raja Permaisuri Bainun • Johor Bahru - Hospital Sultan Ismail • Johor Bahru - Hospital Sultanah Aminah • Klang - Hospital Tengku Ampuan Rahimah • Kota Bharu - Hospital Raja Perempuan Zainab II • Kota Kinabalu - Hospital Queen Elizabeth • Kota Kinabalu - Hospital Wanita dan Kanak-Kanak Sabah • Kuala Lumpur - Hospital Kuala Lumpur • Kuala Terengganu - Hospital Sultanah Nur Zahirah • Kuching - Hospital Umum Sarawak • Melaka - Hospital Melaka • Seremban - Hospital Tuanku Ja'afar • Taiping - Hospital Taiping
Morocco	• Rabat - Adultes - Centre de Référence de l'Hémophilie, Hôpital Ibn Sina • Rabat - Enfants - Centre de Traitement de l'hémophilie de Rabat, Hôpital d'Enfants de Rabat
Nepal	• Kathmandu - Civil Service Hospital
New Zealand	• Christchurch - Christchurch Hospital • Palmerston North - Palmerston North hospital

Country	City-Clinic
Nigeria	• Abuja - National Hospital, Abuja • Benin - University of Benin Teaching Hospital • Enugu State - Southeast HTC, Department of Haematology, UNTH Ituku Ozalla Enugu • Gombe - Gombe State University • Ibadan - University of Ibadan • Kano - Aminu Kano Teaching Hospital • Lagos - Lagos University Teaching Hospital
Pakistan	• Karachi - Haemophilia Welfare Society, Karachi • Lahore - Haemophilia Treatment Centre • Peshawar - Haemophilia Welfare Society, Peshawar • Rawalpindi - Haemophilia Treatment Centre
Panama	• Panamá City - Hospital del Niño
Philippines	• Manila - University of Santo Tomas Hospital
Portugal	• Lisbon - Comprehensive Care Centre of Congenital Coagulopathies, Santa Maria Hospital
Senegal	• Dakar - Centre National de Transfusion Sanguine
Serbia	• Belgrade - Mother and Child Health Care Institute of Serbia “Dr Vukan Cupic”
South Africa	• Bloemfontein - University of the Free State • Groote Schuur Haemophilia Comprehensive care centre • Kimberley - Kimberley Hospital
Sudan	• Khartoum - Haemophilia Center, Khartoum Teaching Hospital
Syria	• Damascus - Syrian Hemophilia Society (SHS)
Thailand	• Bangkok - Department of medicine, Siriraj Hospital • Bangkok - Department of medicine, Thammasat University • Bangkok - Department of paediatrics, Chulalongkorn University • Bangkok - Department of paediatrics, Ramathibodi Hospital • Bangkok - Department of paediatrics, Thammasat University • Chiang Mai - Chiang Mai University Hospital • Nakohn Ratchasima - Department of paediatrics, Maharat Nakohn Ratchasima Hospital • Songkla - Department of paediatrics, Prince of Songkla Universi-ty
Tunisia	• Tunis - Hôpital Aziza Othmana
Uganda	• Kampala - Mulago Hospital
USA	• Winston-Salem - Wake Forest Baptist Health
Venezuela	• Caracas - Centro Nacional de Hemofilia - Banco Municipal de Sangre DC
Vietnam	• Hanoi - National Children's Hospital • Hanoi - National Institute of Hematology and Blood Transfusion • Ho Chi Minh City - Blood Transfusion Hematology
Zambia	• Lusaka - University Teaching Hospital

THANK YOU TO PWH AND PvWD

To each PWH and PwVWD enrolled in the WBDR who has kindly agreed to share their data: thank you for helping improve the quality of care for people with bleeding disorders around the world!

THANK YOU TO HTCs

Thank you to all the dedicated staff at participating hemophilia treatment centers who work hard to ensure that their data meets WBDR data quality standards!

THANK YOU TO SPONSORS

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GRIFOLS

GLOSSARY

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.

Extended half-life factor concentrate: A new generation of recombinant factor concentrates, which extend their half-life. Half-life is the time it takes for infused factor to lose half of its potency. Traditional factor VIII has a half-life of 8 to 12 hours; an extended factor VIII half-life is defined as a ratio greater than 1.3-fold, of the traditional half-life.

Gross National Income: Gross National Income (GNI) per capita (current US$) calculated by The World Bank into four income groups using the Atlas method. The classification is updated each year on July 1st.

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.

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

Mild hemophilia: Condition resulting from a level of factor VIII or factor IX clotting activity above 5% and below 40% of normal activity in the bloodstream. (National definitions differ on the upper limit for mild hemophilia, ranging from 24% to 50%.)

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.

PwVWD: People with von Willbrand Disease.

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.

Standard half-life factor concentrate: Traditional recombinant factor concentrates with a half-life of 8 to 12 hours.

Target joint: Three or more spontaneous bleeds into a single joint within a consecutive 6-month period. Where there have been ≤2 bleeds into the joint within a consecutive 12 month period the joint is no longer considered a target joint[4].

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

REFERENCES

[1] World Federation of Hemophilia. World Bleeding Disorders Registry (WBDR). ClinicalTrials.gov Identifier: NCT03327779. Posted October 31, 2017. https://clinicaltrials.gov/ct2/show/NCT03327779

[2] World Health Organization. 2023. Definition of regional groupings. https://www.who.int/about/who-we-are/regional-offices. Accessed on March 10, 2023.

[3] World Bank 2023. World Bank Data. https://data.worldbank.org/. Accessed October 25, 2023.

[4] Blanchette VS, Key NS, Ljung LR, Manco-Johnson MJ, van den Berg HM, Srivastava A; Subcommittee on Factor VIII, Factor IX and Rare Coagulation Disorders of the Scientific and Standardization Committee of the International Society on Thrombosis and Hemostasis. Definitions in hemophilia: communication from the SSC of the ISTH. J Thromb Haemost. 2014 Nov;12(11):1935-9.

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