WBDR
WFH World Bleeding Disorders Registry

# WFH WBDR
## Kick-off Webinar

December 12, 2017

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

WBDR
WFH World Bleeding Disorders Registry

# TODAY'S GOALS

1 Provide an overview of the WBDR

2 Review important information and processes for participating in the program

3 Offer an opportunity for you to ask questions and/or provide comments

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

# AGENDA

- Background
- Pilot Study
- Global Implementation
  - Program goals
  - Enrollment
  - Privacy
  - Data access
  - Participation processes
- Next steps
- Questions and Feedback

# WFH WBDR TEAM

**Donna Coffin**

**Director, Research & Public Policy**

**Mark Brooker**

**Senior Public Policy Officer**

**Mayss Naccache**

**Data & Research Manager**

**Christine Herr**

**Data & Research Consultant**

**Jennifer Brennan**

**Research & Public Policy Assistant**

## POLLING QUESTIONS

Do you represent:

- WFH National Member Organization
- Hemophilia Treatment Centre
- Person with hemophilia
- Interested stakeholder

# POLLING QUESTIONS

Did you participate in the WBDR Pilot Study?

- Yes
- No

WBDR
WFH World Bleeding
Disorders Registry

# POLLING QUESTIONS

What region of the world are you from?

- Africa
- Americas
- Eastern Mediterranean
- Europe
- South-East Asia
- Western Pacific

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

## POLLING QUESTIONS

Have you already applied to participate in the WBDR?

- Yes
- No

# INTRODUCTORY REMARKS

**Jamie O’Hara**

**Member, WBDR Steering Committee**

# WBDR STEERING COMMITTEE MEMBERS

- **Glenn Pierce*, MD, PhD, USA, Chair**
- Alok Srivastava, MD, India, Member
- David Lillicrap, MD, Canada, Member
- Jamie O’Hara*, MSc, UK, Member
- Rob Hollingsworth, PhD, UK, Member
- Saliou Diop, MD, Senegal, Member
- Mike Soucie, PhD, CDC, Member
- Cedric Hermans, MD, Belgium, Member
- Craig Upshaw*, MBA, Canada, Member
- Alfonso Iorio, MD, Canada, Member
- Marijke van den Berg, MD, Netherlands, VP Medical, Ex-officio
- Donna Coffin, MSc, WFH
- Christine Herr, MPH, WFH
- Mayss Naccache, MSc, WFH

*patient representatives

# VALUE OF INTERNATIONAL REGISTRIES TO THE BLEEDING DISORDER COMMUNITY

WBDR
WFH World Bleeding Disorders Registry

Standardized Data Collection

Increase Standards of Care

Research

Advocacy

Data = Evidence

**Patient/Population Levels**

No registry=
No data

No data=
No advocacy

No advocacy=
No improved treatment

No improved treatment
= Status quo

## PILOT STUDY

# World Bleeding Disorders Registry
# Pilot Study
# April – December 2016

WBDR
WFH World Bleeding Disorders Registry

# PILOT STUDY RESULTS

## WORLD BLEEDING DISORDERS REGISTRY

*Pilot Study, April–December 2016*

**26** Hemophilia Treatment Centres participated representing **25 countries**

**356** people with hemophilia enrolled

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

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

WBDR
WFH World Bleeding Disorders Registry

# PILOT STUDY LEARNINGS

- Feedback from HTC pilot participants was very positive overall
- Found there was great interest and ability from HTCs, ethics committees and patients worldwide to successfully participate in a web-based patient registry

**Issues Identified by HTCs**

- Obtaining ethics approval
- Limited staff resources
- Language options
- Technical difficulties

**Issues Resolved by WFH**

- Allowed additional time for ethics review
- Allowed additional time for resourcing
- Made multi-language options available
- Solved technical issues and made enhancements

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

WBDR
WFH World Bleeding Disorders Registry

# FULL-SCALE GLOBAL LAUNCH

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

# WBDR IS LAUNCHING GLOBALLY

The WBDR is the only global registry collecting standardized clinical data on people with hemophilia from around the world

WBDR
WFH World Bleeding
Disorders Registry

The WBDR could ultimately improve the quality and access to care for all people living with hemophilia

# WHAT IT IS

## Format:

Privacy-protected online web-based data entry system that will provide a platform for a network of HTCs around the world to collect data

## Objective:

Develop a global database of high-quality, real world data on a large population of people with hemophilia

## Study Design:

Prospective, longitudinal and observational registry of patients with hemophilia A & B

WBDR
WFH World Bleeding
Disorders Registry

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

# GLOBAL COMMUNITY INVOLVEMENT

***Everyone plays an active part in building this important resource***

The success of the WBDR depends on the valuable involvement and support from HTCs, NMOs and people with hemophilia from around the world

It presents an opportunity to contribute data to advance the understanding and care of people with hemophilia worldwide and support evidence-based advocacy initiatives

# DATABASE DEVELOPMENT

Karolinska Institutet
ANNO 1810

Karolinska
Institutet

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

HealthSolutions

***Initial funding has been confirmed for the launch of the full-scale WBDR***

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

# PATIENT ELIGIBILITY AND ENROLLMENT

- At this time, the WBDR is available to:
  - Patients diagnosed with **hemophilia A or B**, who
  - **Receive care through participating HTCs** in countries around the world

- Participating HTCs are encouraged to **take opportunities before or during clinic visits** to talk to each of their hemophilia patients **about being enrolled** into the WBDR registry
  - A written form seeking informed consent can then be given to interested patients for signing

# PATIENT DATA PRIVACY AND PROTECTION

**Standards are in place:**

- Personal data will not be entered into the database; only de-identified data will be entered into the database
- The database will adhere to the European Union's strict patient data privacy and protection laws
- The database will be based in Sweden and will adhere to CE-mark (Conformité Européenne) and the UK standard IGSoc (Information Governance Standard of Compliance)
- The database will be compliant with new 2018 Program General Data Protection Regulation (GDPR) in the EU, which will come into effect in May 2018

# DATABASE TIMELINE

**Minimal Data Set**

- Target date: end of January 2018
- Field testing with 10 sites

**Extended Data Set**

- Development begins January 2018
- Target date: mid-2018

**Patient App + PRO**

- 2018 project
- Target date: end of 2018

# MINIMAL DATA SET

- **Demographics**
  - Date of birth
  - Gender
  - Country of residence

- **Diagnostics**
  - Date of diagnosis
  - Hemophilia type, severity, factor level
  - Disease history

- **At each follow-up clinic visit**
  - Bleeding events
  - Target joints
  - Treatments
  - Inhibitor status
  - Hospitalization
  - Mortality

# UNIQUE PATIENT IDENTIFIER

- A unique patient identifier (UPI) will be computer-generated for each participating patient
- This UPI will be provided to the participating patient on a WBDR Identification Card
- UPI will detect duplicate patients
- UPI will follow patients if they transfer to another participating HTC

# ENROLLMENT GOALS TO MEET AND EXCEED – 5-YEAR

**50**

Countries

**200**

HTCs

**10K**

PWH

# ENROLLMENT GOALS - 2018

**50**

Countries

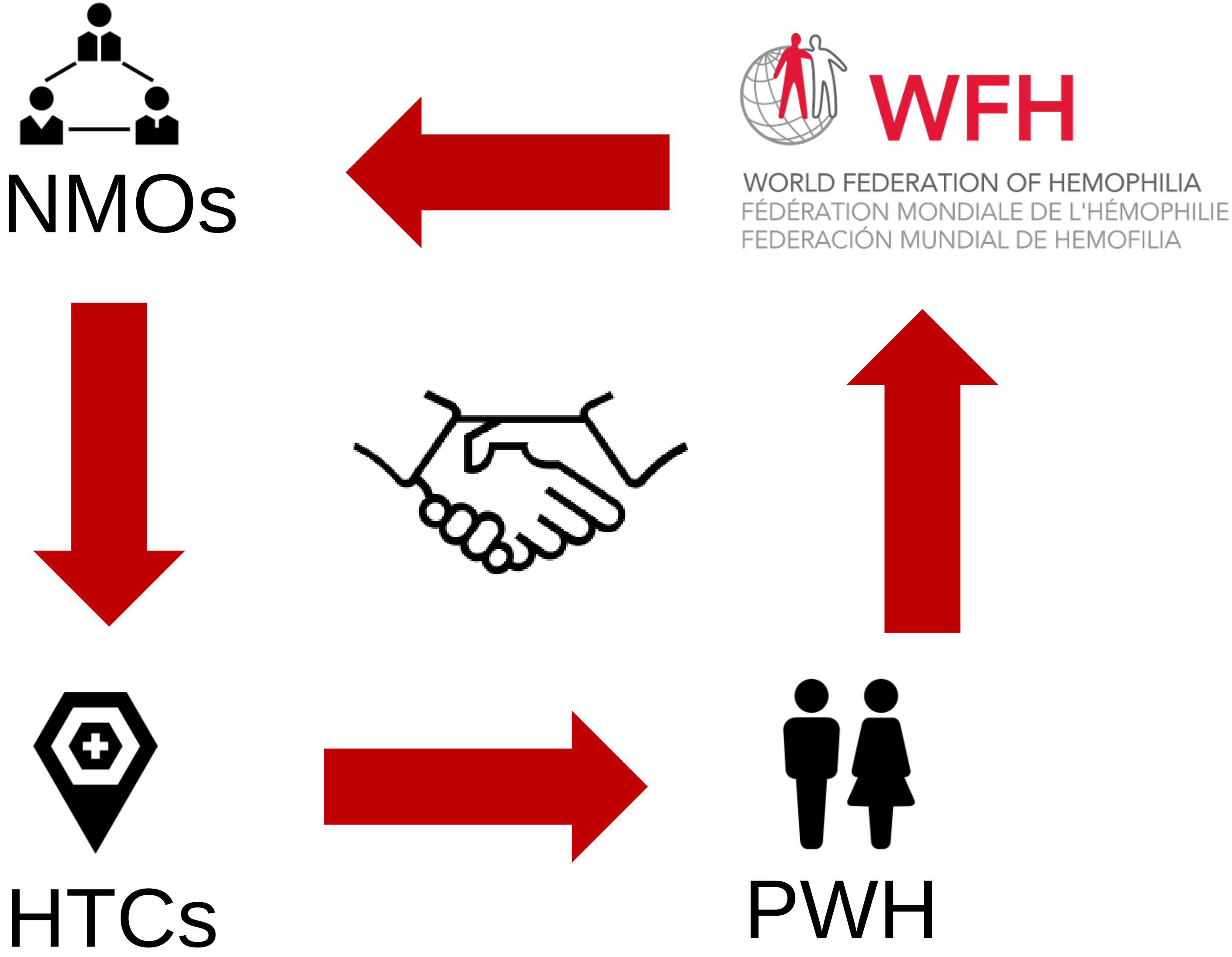
**75**

HTCs

**1K**

PWH

# WORKING TOGETHER TO ACHIEVE GOALS



# IMPLEMENTATION

## Process

**NMO** — **HTC** — **PWH**

Information Letter

Invitations

Informed Consent

# IMPLEMENTATION

## Process

**NMO**

✓ Information Letter

**HTC**

Invitations

**PWH**

Informed Consent

Oct – Nov 2017

**134 countries**

# IMPLEMENTATION

## Process

**NMO** — **HTC** — **PWH**

✓ Information Letter

✓ Invitations

Informed Consent

Oct – Nov 2017
**134 countries**

Nov – Dec 2017
**~ 200 HTCs**

# IMPLEMENTATION

## Process

**NMO** — **HTC** — **PWH**

✓ Information Letter

Oct – Nov 2017

**134 countries**

✓ Invitations

Nov – Dec 2017

**~ 200 HTCs**

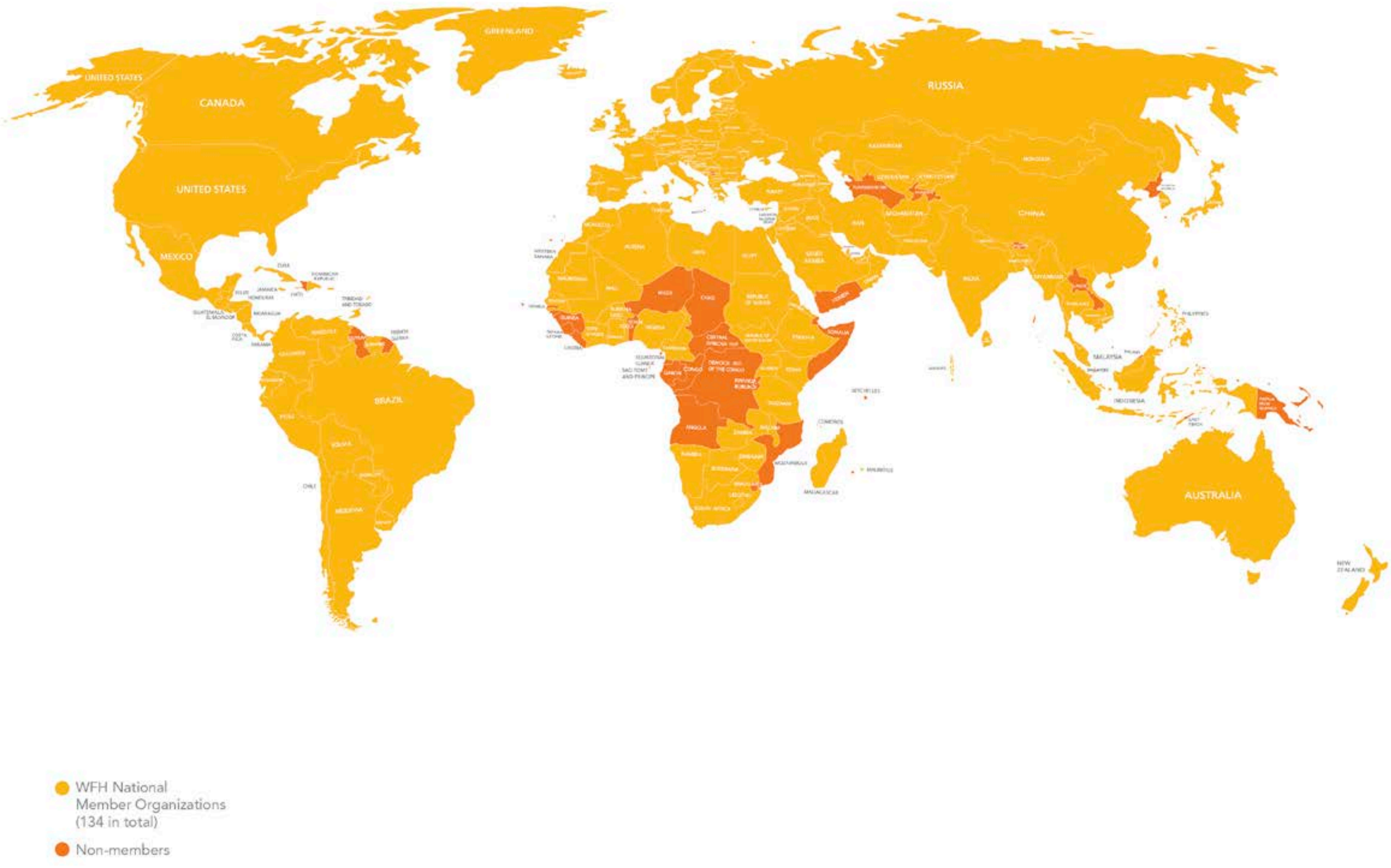
Informed Consent

January 2018

**1st Patient**

WBDR
WFH World Bleeding Disorders Registry

# WBDR COUNTRIES



## Global reach to a network of >1000 HTCs and WFH NMOs in 134 countries

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

# DATA LINKAGE STRATEGY

Countries with an established patient registry, who are interested in linking with the WBDR, will require a data linkage strategy.

*In development*

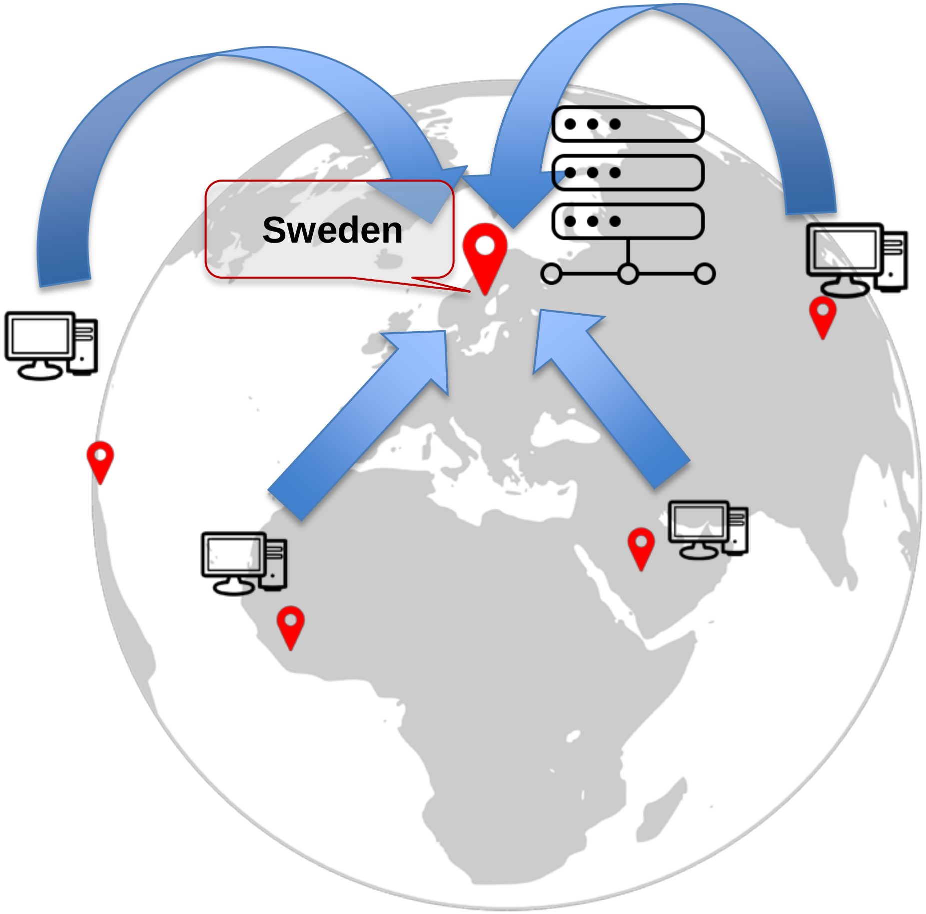
# DATA MANAGEMENT PLAN

- Data Validation Plan
  - Automated edit-checks and logical checks
  - Automated missing values check
  - Patient summary review by HTCs

- Regional Managers

# DATA FLOW IN THE WBDR

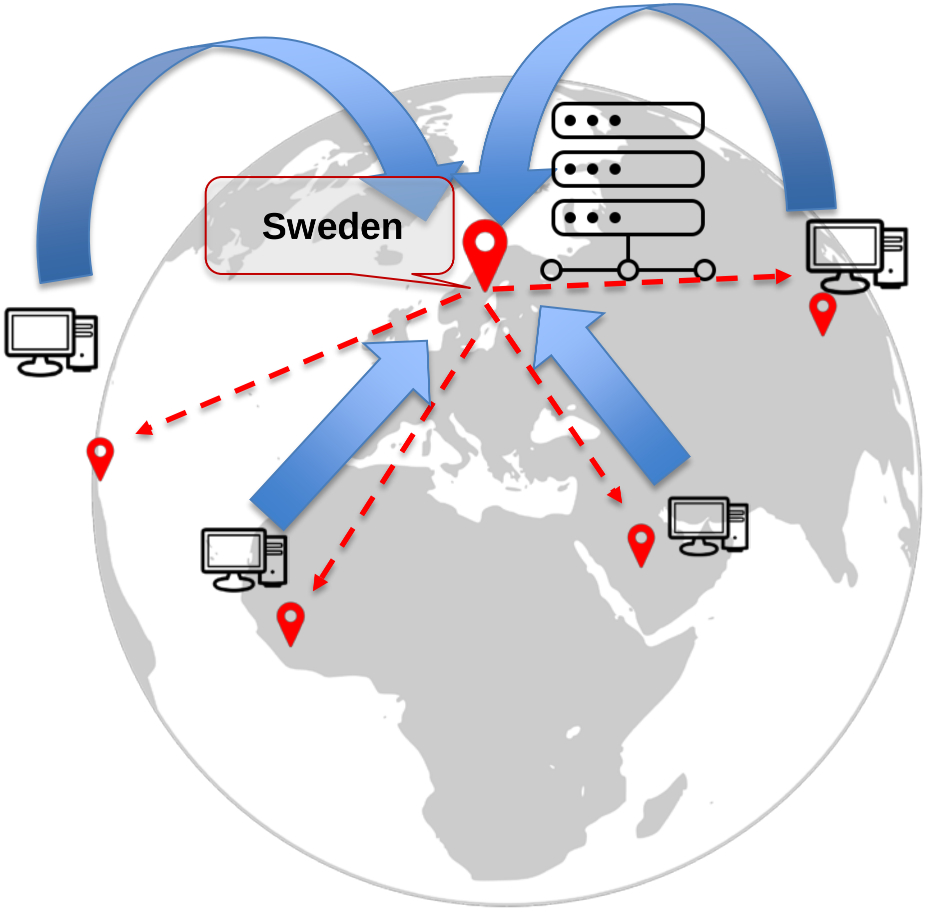
Data flows from HTCs around the globe into one central database in Sweden



# ACCESSING YOUR HTC DATA

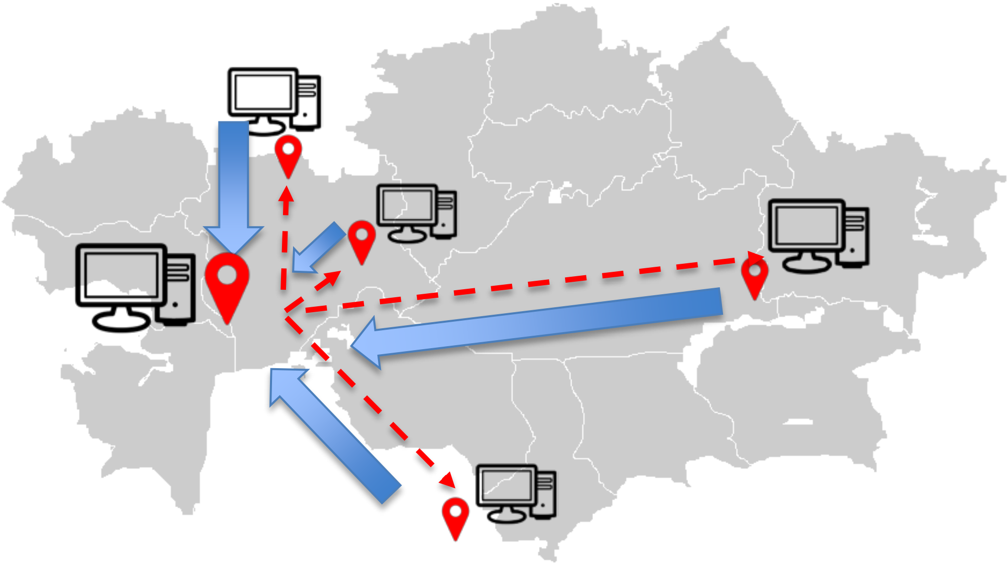
Each HTC can view and use data from their HTC only

No one can view data from other HTCs



# DATA SHARING AGREEMENTS

Through a Data Sharing Agreement, data from HTCs in the same country can be combined, thus providing country-wide data



# RESEARCH GOVERNANCE

- WBDR Research Committee

- To guide scientific utilisation of registry data, assess external applications for utilisation of data, encourage data dissemination

# HIGH-LEVEL WBDR STEPS FOR HTCS

1. Ensure HTC criteria are met
2. Obtain Ethics approval
3. Receive WBDR database training
4. On ongoing basis, talk to each eligible patient to obtain informed consent
5. On ongoing basis, input patient data into database

WBDR
WFH World Bleeding Disorders Registry

# HTC REQUIREMENTS

Criteria for HTC participation encourages the **uniform collection of standardized patient data**, ultimately allowing researchers to use country-specific data and comparative data to **generate evidence and build advocacy initiatives** aimed at health policy decision-makers

**As the WBDR is a long-term, web-based data entry system, HTC participation criteria include:**

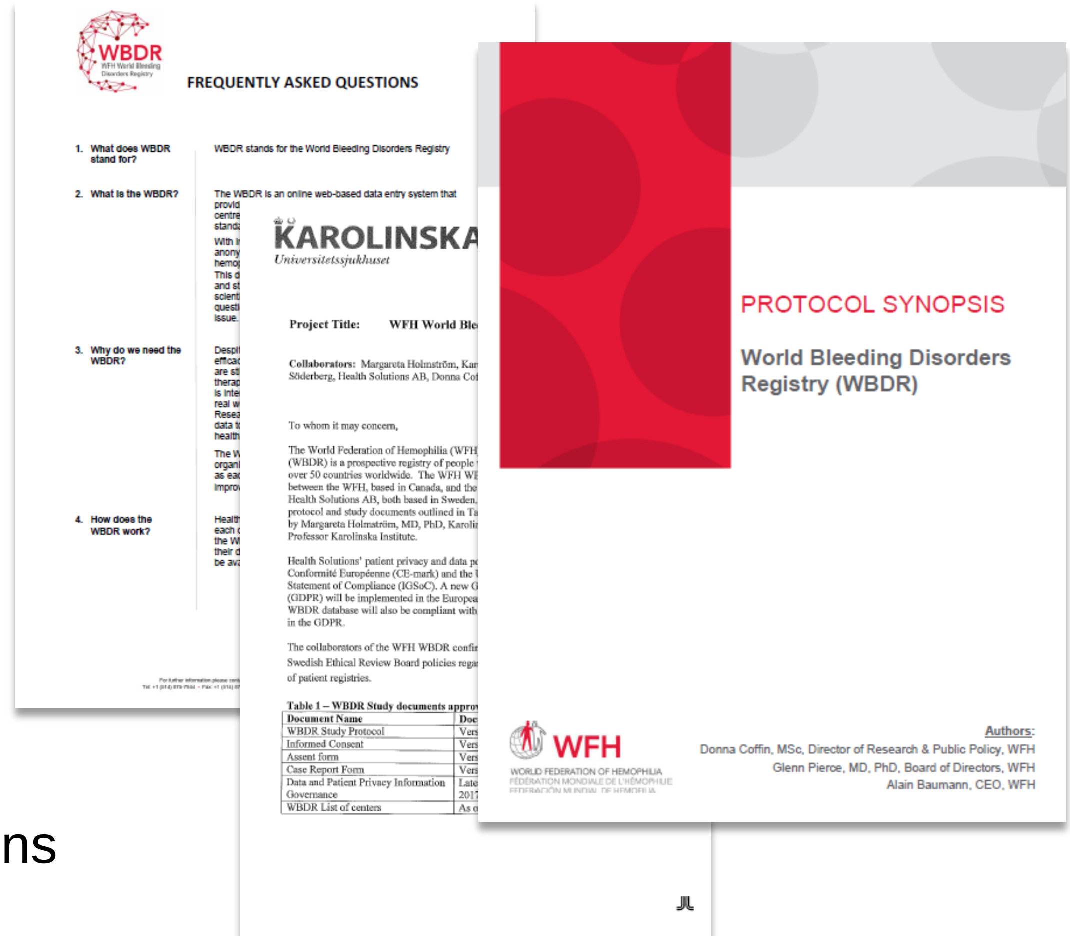
1. Completed WBDR HTC Application Form
2. Reliable access to the internet
3. Consecutive enrollment
4. Long-term commitment
5. Ethics approval
6. Informed patient consent
7. Data entry
8. Language

# ETHICS APPROVAL PROCESS

- Date of the next Ethics Committee or Institutional Review Board meeting
- Deadline to submit required documents
- Documents and forms required for your submission

# ETHICS SUBMISSION

- WBDR Ethics Approval Letter
- Final Protocol
- Consent Form
- Assent Form
- Data Collection Tools:
  1. HTC Registration Form
  2. Minimal Data Set
- Introduction Letter
- Frequently Asked Questions



FREQUENTLY ASKED QUESTIONS

1. What does WBDR stand for? — WBDR stands for the World Bleeding Disorders Registry
2. What is the WBDR? — The WBDR is an online web-based data entry system that
3. Why do we need the WBDR?
4. How does the WBDR work?

KAROLINSKA
*Universitetssjukhuset*

**Project Title:** **WFH World Ble**

**Collaborators:** Margareta Holmström, Kar
Söderberg, Health Solutions AB, Donna Cof

To whom it may concern,

The World Federation of Hemophilia (WFH
(WBDR) is a prospective registry of people
over 50 countries worldwide. The WFH WE
between the WFH, based in Canada, and the
Health Solutions AB, both based in Sweden,
protocol and study documents outlined in Ta
by Margareta Holmström, MD, PhD, Karolin
Professor Karolinska Institute.

Health Solutions' patient privacy and data p
Conformité Européenne (CE-mark) and the
Statement of Compliance (IGSoC). A new G
(GDPR) will be implemented in the Europea
WBDR database will also be compliant with
in the GDPR.

The collaborators of the WFH WBDR confi
Swedish Ethical Review Board policies rega
of patient registries.

**Table 1 – WBDR Study documents appro**

| **Document Name** | **Doc** |
|---|---|
| WBDR Study Protocol | Vers |
| Informed Consent | Vers |
| Assent form | Vers |
| Case Report Form | Vers |
| Data and Patient Privacy Information Governance | Late<br>2017 |
| WBDR List of centers | As o |

PROTOCOL SYNOPSIS

World Bleeding Disorders Registry (WBDR)

Authors:
Donna Coffin, MSc, Director of Research & Public Policy, WFH
Glenn Pierce, MD, PhD, Board of Directors, WFH
Alain Baumann, CEO, WFH

# ETHICS SUPPORT

WFH can help with:

- Modifying and providing required documents
- Translation into local language

# WHAT'S TO COME: WFH PROVIDES ONGOING SUPPORT, UPDATES AND TRAINING

WBDR
WFH World Bleeding Disorders Registry

- HTC Tool Kit
- Online Portal
- Patient Discussion Program Tools
- Database Training Material
- Training & Program Update Webinars
- In-Person Trainings
- Program Update E-Newsletter Series

***Are there any other tools you might need to help you feel successful in your program participation and patient discussions?***

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

# WHAT'S TO COME: EXAMPLE OF PATIENT DISCUSSION PROGRAM TOOLS

WBDR
WFH World Bleeding Disorders Registry

WBDR
WFH World Bleeding Disorders Registry

**JOIN**
THE WFH WORLD BLEEDING DISORDERS REGISTRY (WBDR) **TODAY!**

**BY PARTICIPATING,** you can help shape the future care of people with hemophilia around the world!

The WBDR is a secure and confidential global registry of people with hemophilia A and B.

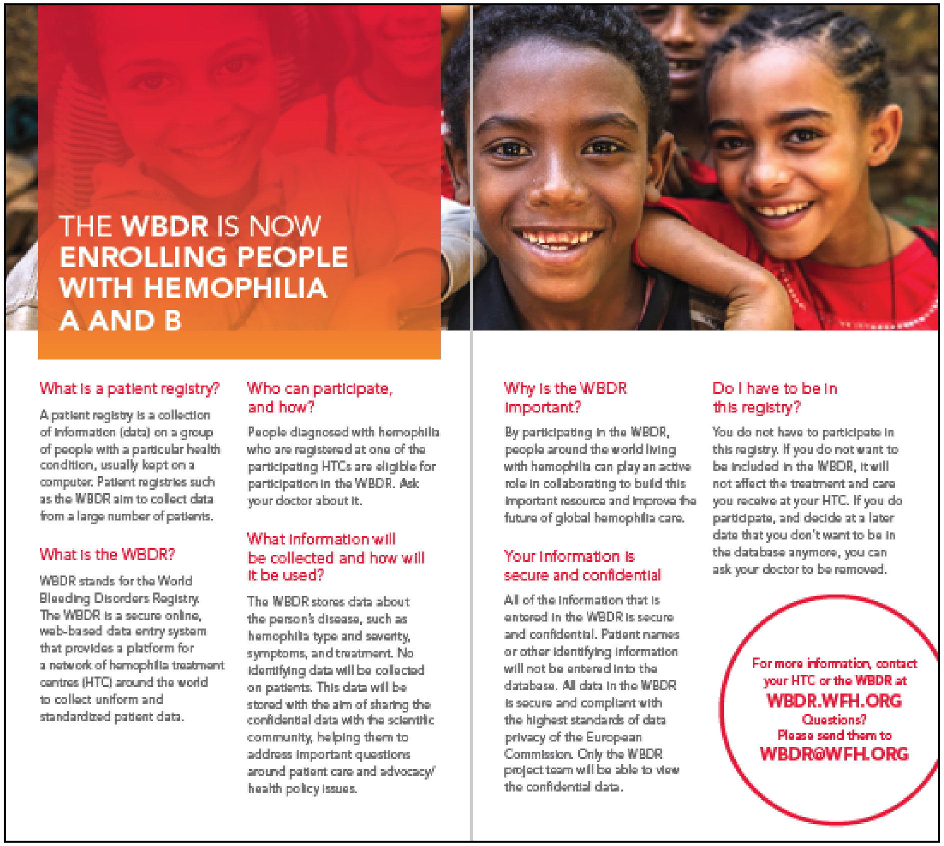
THE WBDR IS **NOW ENROLLING PEOPLE WITH HEMOPHILIA A AND B**

For more information ask your doctor about the WBDR or contact us at **WBDR.WFH.ORG**

THE WFH THANKS THE FOLLOWING PARTNERS FOR THEIR VALUABLE SUPPORT OF THE WBDR:

| Visionary Partners | Collaborating Partners |
|---|---|
| Shire<br>Sobi | Bayer<br>F. Hoffman-La Roche Ltd.<br>Grifols<br>Pfizer<br>Sanofi Genzyme |

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



THE WBDR IS NOW **ENROLLING PEOPLE WITH HEMOPHILIA A AND B**

**What is a patient registry?**

A patient registry is a collection of information (data) on a group of people with a particular health condition, usually kept on a computer. Patient registries such as the WBDR aim to collect data from a large number of patients.

**What is the WBDR?**

WBDR stands for the World Bleeding Disorders Registry. The WBDR is a secure online, web-based data entry system that provides a platform for a network of hemophilia treatment centres (HTC) around the world to collect uniform and standardized patient data.

**Who can participate, and how?**

People diagnosed with hemophilia who are registered at one of the participating HTCs are eligible for participation in the WBDR. Ask your doctor about it.

**What information will be collected and how will it be used?**

The WBDR stores data about the person's disease, such as hemophilia type and severity, symptoms, and treatment. No identifying data will be collected on patients. This data will be stored with the aim of sharing the confidential data with the scientific community, helping them to address important questions around patient care and advocacy/ health policy issues.

**Why is the WBDR important?**

By participating in the WBDR, people around the world living with hemophilia can play an active role in collaborating to build this important resource and improve the future of global hemophilia care.

**Your information is secure and confidential**

All of the information that is entered in the WBDR is secure and confidential. Patient names or other identifying information will not be entered into the database. All data in the WBDR is secure and compliant with the highest standards of data privacy of the European Commission. Only the WBDR project team will be able to view the confidential data.

**Do I have to be in this registry?**

You do not have to participate in this registry. If you do not want to be included in the WBDR, it will not affect the treatment and care you receive at your HTC. If you do participate, and decide at a later date that you don't want to be in the database anymore, you can ask your doctor to be removed.

For more information, contact your HTC or the WBDR at
**WBDR.WFH.ORG**
Questions?
Please send them to
**WBDR@WFH.ORG**

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

# WHAT'S TO COME:
# WFH 2018 WORLD CONGRESS

THE LARGEST INTERNATIONAL
**MEETING FOR THE GLOBAL**
**BLEEDING DISORDERS COMMUNITY**

GLASGOW, SCOTLAND • MAY 20-24, 2018

ORGANIZED BY: WORLD FEDERATION OF HEMOPHILIA
HOSTED BY: THE HAEMOPHILIA SOCIETY

WBDR

WFH World Bleeding Disorders Registry

# Questions?
# Feedback?

WBDR

WFH World Bleeding Disorders Registry

# THANK YOU

**wbdr@wfh.org**

***Please contact us with questions***