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

GTR
WFH GENE THERAPY REGISTRY

WFH Gene Therapy Registry

User guide for people with hemophilia

World Federation of Hemophilia
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The World Federation of Hemophilia (WFH) Gene Therapy Registry (GTR) is a private and secure database that collects clinical information about people with hemophilia (PWH) who receive gene therapy, whether in a clinical trial or as an approved product.

The WFH GTR is available to access by all PWH globally through their Hemophilia Treatment Centre (HTC).

Not all PWH will be eligible to receive gene therapy, meaning that the number of people enrolling in this registry will be relatively small. Therefore, the data from each participant treated with gene therapy are essential. Your involvement in the WFH GTR will help advance our knowledge and understanding of gene therapy for PWH, improve clinical care, and develop the next generation of gene therapy treatments.

The WFH GTR was developed by international experts in gene therapy and hemophilia.

Working Together with your Hemophilia Treatment Centre

The health care team at your HTC will work closely with you and your family/caregivers throughout your participation in the WFH GTR. They will provide you with the information you need to get started in the registry and answer any questions you may have. They serve as the main point of contact for anything related to your participation in the registry.

Before joining the registry, you should communicate openly with your health care team at your HTC and make sure all of your questions are answered. The health care team will enter your clinical data into the registry at each of your scheduled clinic appointments. You will not have any additional tests or visits based solely on your participation in the WFH GTR.

Participation and Protections

Participation in the WFH GTR is voluntary. If you decide to join the registry, you can stop at any time without question. Your treatment and follow-up care will not be affected by your decision to either participate in or leave the registry. You will continue to receive the same standard of care regardless of your decision and have regularly scheduled clinic visits with your HTC to monitor potential side effects and how well your treatment is working.

An independent committee called an Institutional Review Board (IRB) protects the rights and welfare of participants involved in the registry. It ensures that all research conducted is held to the highest ethical standards. Your HTC will obtain approval from their local IRB before enrolling participants.

Data Privacy and Security

The WFH GTR is highly secure and compliant with the highest security standards. Data entered in the registry database are de-identified to protect your privacy, meaning that your identity, and that of any other individual enrolled in the registry, is protected. Participant names or other identifying information will not be stored in the database.

Joining the WFH GTR

When you have made an informed decision to receive gene therapy (in partnership with your health care team), you will be invited to participate in the registry. If you have already received gene therapy through a clinical trial, you will also be invited to join.

All PWH who have received gene therapy can participate in the registry regardless of where they are in their gene therapy journey, whether at the infusion stage or a few years since.

Providing Informed Consent

If you decide to participate in the registry, you will be asked to review and sign an informed consent form. This states that you understand the benefits and risks of participating in the WFH GTR and agree to participate. Your health care team has been trained to provide you with the necessary information to help you make an informed decision about participation in the registry.

Your health care team will also ensure that you have an appropriate amount of time to ask questions and discuss with your family/caregivers as to whether you should participate. Your HTC will continue to provide you with information regarding any changes to the registry during your participation. If you decide to participate, you and your physician will be asked to sign the consent form.

SCAN QR CODE to access the WFH document **Questions to Ask Before Participating in a Clinical Trial**

Before signing the consent form, your health care team will work with you to ensure you understand the following:

- The purpose of the registry
- The responsibilities of participants
- The benefits and risks of participation
- That participation is entirely voluntary and can end at any time
- The expected duration of participation
- The contact information for your HTC

Data Collection

Data will be collected and entered into the WFH GTR database at your regularly scheduled clinic visits. You will not have any additional clinic visits, tests, or procedures because of your participation. The registry will collect the same information from all participants who have received gene therapy.

Baseline Data

Your HTC will collect demographic data after you agree to join the registry. This includes information on your hemophilia diagnosis and medical history.

Gene Therapy Infusion

Details regarding your gene therapy treatment, including date and infusion information, will be entered into the registry.

Follow-up Visits

After you receive gene therapy, your HTC will collect data every time you come for a visit. This includes several visits during the first two years to determine how well the treatment is working, ensuring your health and safety, and at least annually thereafter.

Participating in the registry will not affect your care or clinic visit schedule.

Safety and Adverse Event Reporting

As with any new medication or procedure, you need to report all health events to your HTC. At each clinic visit, your health care team will ask you about any side effects you may have experienced. This information will be collected in the registry for all participating PWH. By gathering this data on all registry participants from around the world, doctors will be able to identify any common or serious side effects related to the gene therapy.

Patient-reported Outcomes

In addition to the medical data collected at each visit, you will have the opportunity to provide information directly into the registry from your phone via a mobile application ("myGTR"). Data that you enter directly are called patient-reported outcome (PRO) data. This includes information on your quality of life, how your condition affects you, and any bleeding events or treatment you may receive.

PRO data are important because they allow you to provide your own experiences and perspectives into the registry. The data you enter in myGTR will be directly transmitted to the WFH GTR database at your HTC, and your physician will be able to see them. You will also receive output data in the application on your phone, allowing you to monitor your progress over time.

Languages

Initially, the registry will be available in English, with plans to translate it into other languages as needed. The mobile patient app, myGTR, will also be available in English and other languages when required.

Length of Participation in the Registry

The long-term data collected in the registry is important for yourself and your peers because it will contribute to the global hemophilia community's understanding of gene therapy. Your continued retention over the long-term in the registry is important. However, you are free to stop participating in the registry at any time, for any reason – simply speak to your health care team to let them know. If there are major changes in the WFH GTR, your health care team will contact you, and you may be asked to sign a new consent form.

Accessing Data in the WFH GTR

You can access your data at any time by talking to your health care team. Members of your health care team will also be able to view and access your data in the registry. The WFH and database provider will be able to view all anonymized data entered in the registry. The WFH GTR Scientific Advisory Board governs the data in the registry and will evaluate and approve any data request (**see appendix A**).

Researchers, manufacturers, and other interested stakeholders may submit a request to the WFH to access de-identified data to examine trends and answer research questions. Manufacturers will have access to de-identified data to examine product-specific safety and efficacy outcomes. By participating in this registry, you will contribute to the development of the next generation of gene therapy treatments.

Best Practices for PWH Participating in the WFH GTR

- Write down your questions ahead of time and bring them to your clinic visits.
- If you don't understand something, just ask! The research or clinical team expects that you will have questions for them, so feel free to ask as many as you need to.
- Make sure to attend all HTC visits. These are essential for monitoring your health and safety and improving the outcomes of gene therapy for future generations.
- Keep communication lines open with your health care team. Talk with them about your health concerns and other barriers you might face in your follow-up care.
- Use the myGTR mobile app!

Appendix A

WFH GTR Scientific Advisory Board

World Federation of Hemophilia; Medical Board member (chair of WFH GTR Steering Committee)

World Federation of Hemophilia; Vice President-Medical

National Hemophilia Foundation; Medical and Scientific Advisory Council representative

European Haemophilia Consortium; Medical Advisory Group representative

European Association for Haemophilia and Allied Disorders; representative

International Society on Thrombosis and Haemostasis; representative

Patient advocates (2)

The GTR is supported by funding from:

FOUNDING VISIONARY PARTNERS

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Notes

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