



GTR

WFH GENE THERAPY
REGISTRY

WFH Gene Therapy Registry

**ENHANCING OUR KNOWLEDGE
OF GENE THERAPY AND
KEEPING PATIENTS SAFE**

The WFH Gene Therapy Registry (GTR) is a global initiative aimed at collecting long-term data on people with hemophilia (PWH) who receive gene therapy. These data will enhance our understanding of gene therapy and ensure the hemophilia community has access to timely evidence on the long-term safety and efficacy of gene therapy.



WFH

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



The need for ensuring the safety of the community

As gene therapy products for hemophilia are approved, it is imperative that we consider the long-term impacts on patients. The number of people with hemophilia who receive gene therapy may be limited at first—and dispersed over many countries—making it a challenge to identify outcome patterns and compare individual patient outcomes in a meaningful way. By pooling data from all countries, the WFH GTR will make it easier to perform robust evaluation of safety events and establish the durability of gene therapy. In this way, the registry is the best way to ensure that rare adverse events are detected, even in smaller and geographically dispersed populations.

GET INVOLVED!

The safety of patients with hemophilia is a shared responsibility. All people with hemophilia who have received, or who will receive, gene therapy in the future can participate in the WFH GTR regardless of where they are in their treatment process. The WFH encourages all HTC and national hemophilia registries to join this global initiative.

How the WFH GTR works

The WFH GTR is an online web-based system that collects uniform and standardized data on all PWH who receive gene therapy, either via a clinical trial or via a gene therapy product post-approval. The core dataset of the GTR was developed by a multi-stakeholder steering committee and is available online. Patient data will be directly inputted by hemophilia treatment centres (HTC) participating with the GTR, and added through our Gene Therapy Data Integration Program, which allows existing national registries to link directly with the WFH GTR, thus eliminating data entry duplication.

Contact

For more information on the WFH GTR, please visit wfh.org/data-collection. If you are a patient and you wish to participate in the WFH GTR, please contact your local HTC. If you are with an HTC or a national registry, and wish to participate, please contact the WFH at gtr@wfh.org.

Our partners

The WFH GTR was developed in collaboration with the American Thrombosis and Hemostasis Network (ATHN), the European Association for Haemophilia and Allied Disorders (EAHAD), the European Haemophilia Consortium (EHC), the International Society of Thrombosis and Hemostasis (ISTH), the National Hemophilia Foundation (NHF), industry gene therapy development partners and regulatory liaisons.

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Founding visionary partners

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