

## World Federation Hemophilia Gene Therapy Registry Governance

The WFH Gene Therapy Registry (GTR) has established a governance structure to ensure the continued success of the program (Figure 1).

### **Scientific Advisory Board**

The role of the WFH GTR Scientific Advisory Board (SAB) is to provide expert scientific advice to the WFH regarding the WFH GTR, particularly focusing on the data charter, research policies, and scientific issues. Representatives on the SAB are nominated by their respective organizations. Each representative can represent only one organization. Membership will be for a duration of up to 4 years.

### **National Registries & Hemophilia Treatment Centres Consortium**

The role of the National Registries & Hemophilia Treatment Centres Consortium is to provide a platform for collaboration, information and idea exchange on data usage, exchange, standardization, privacy, etc. The National Registries & Hemophilia Treatment Centres Consortium is a sub-committee of the SAB. Representatives on the Consortium are nominated by their respective organizations/country. Each representative can represent only one organization on the Consortium.

### **Steering Committee**

The role of the WFH GTR Steering Committee (SC) is to provide leadership, oversight, and expert advice to the WFH regarding the GTR, particularly focusing on the development, implementation, and structure of the GTR. Representatives on the SC are nominated by their respective organizations. Each representative can represent only one organization. Membership will be for a duration of up to 4 years.

### **Industry Consortium**

The role of the Industry Consortium is to provide insights into data needs, post-approval surveillance and long-term data needs.

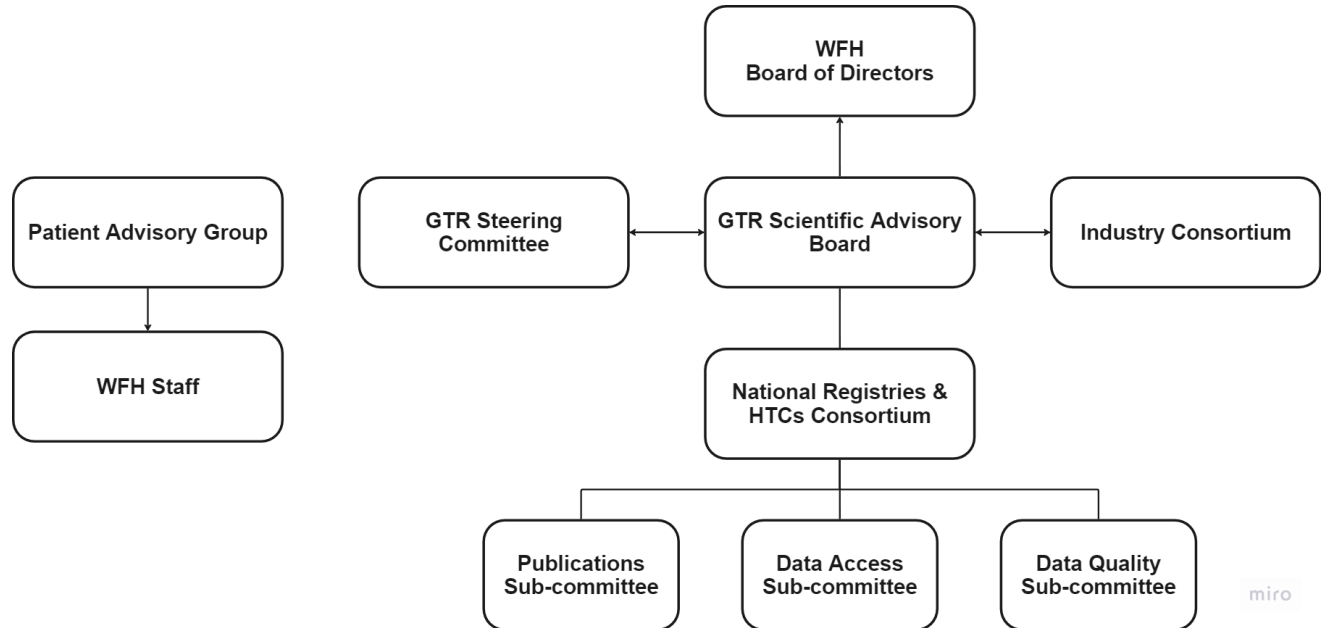
### **GTR Patient Advisory Group**

The role of the GTR Patient Advisory Group provides guidance on all patient related GTR initiatives.

Meetings are held on a regular basis, and on an ad hoc basis if necessary. All decisions are made by discussion and consensus. The WFH Board of Directors retains the right of approval or veto all decisions and helps resolve issues that cannot be resolved by the Scientific Advisory Board.

Details of the members from the different governance groups can be found in Appendix A.

**Figure 1. WFH Gene Therapy Registry governance**



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## Appendix A

### WFH GTR Scientific Advisory Board members

Representation	Name	Affiliation
Co-chair, WFH Board of Directors member	Barbara Konkle	University of Washington, Seattle, United States
Co-chair, ISTH	Flora Peyvandi	Università degli Studi di Milano, Milan, Italy
VP Medical, WFH	Glenn Pierce	WFH
EAHAD	Wolfgang Miesbach	University Hospital Frankfurt, Frankfurt, Germany
EHC Medical Advisory Group	Maria Elisa Mancuso	University of Sheffield, Sheffield, United Kingdom
NHF MASAC	Amy Dunn	Nationwide Children's Hospital, Columbus, United States
Patient advocate	Brian O'Mahony	Irish Haemophilia Society
Patient advocate	Mark Skinner	Institute for Policy Advancement Ltd

### WFH GTR National Registries & Hemophilia Treatment Centres Consortium

Representation	Name	Affiliation	
SAB Co-chair, WFH Board of Directors member	Barbara Konkle	University of Washington, Seattle, United States	
SAB Co-chair, ISTH	Flora Peyvandi	Università degli Studi di Milano, Milan, Italy	
VP Medical, WFH	Glenn Pierce	WFH	
Country	Registry/HTC	Name	Affiliation
Australia	Registry	Chris Barnes	Royal Children's Hospital, Melbourne, Australia
Canada	Registry	Alfonso Iorio	McMaster University, Hamilton, Canada
France	Registry	Herve Chambost	
Germany	Registry	Andreas Tiede	
Germany	Registry	Christine Keipert	Paul-Ehrlich-Institut, Division of Haematology / Transfusion Medicine, Langen, Germany
Japan	Registry	Takeshi Matsumoto	Mie University Hospital, Tsu, Japan
Netherlands	Registry	Samantha Gouw	Academic Medical Centre Amsterdam, Amsterdam, The Netherlands
UK	Registry	Pratima Chowdary	University College London, London, UK
USA	Registry	Tami Singleton	Tulane University, New Orleans, USA

Belgium	HTC	Cedric Hermans	Cliniques universitaires Saint-Luc, Brussels, Belgium
Brazil	HTC	Margareth Castro Ozelo	University of Campinas, São Paulo, Brazil
Ireland	HTC	Niamh O'Connell	St. James's Hospital, Dublin, Ireland
Saudi Arabia	HTC	Hazza El Zahrani	King Faisal Specialist Hospital & Research Centre, Riyadh, Saudi Arabia
South Africa	HTC	Johnny Mahlangu	University of the Witwatersrand, Johannesburg, South Africa
Spain	HTC	Santiago Bonanad	Hospital Universitari i Politècnic La Fe, Valencia, Spain
Sweden	HTC	Jan Astermark	Lund University, Malmö, Sweden

#### WFH GTR Steering Committee members

Representation	Name	Affiliation
Chair, WFH Board of Directors member	Barbara Konkle	University of Washington, Seattle, United States
WFH VP Medical	Glenn Pierce	WFH
EAHAD	Wolfgang Miesbach	University Hospital Frankfurt, Frankfurt, Germany
ISTH	Cary Clark	ISTH
ISTH	Johnny Mahlangu	University of the Witwatersrand, Johannesburg, South Africa
ISTH	Flora Peyvandi	Università degli Studi di Milano, Milan, Italy
NHF, MASAC	Amy Dunn	Nationwide Children's Hospital, Columbus, United States
Leader in the field, University of Michigan	Steve Pipe	University of Michigan, Ann Arbor, United States
Leader in the field, Children's Hospital of Philadelphia	Lindsey George	The Children's Hospital of Philadelphia, Philadelphia, United States
Patient advocate, EHC	Miguel Crato	EHC
Patient advocate, coreHEM, PROBE	Mark Skinner	Institute for Policy Advancement Ltd
Industry - Biomarin	Carole Paley	Biomarin
Industry - Pfizer	Lisa Wilcox	Pfizer

Industry - Spark	Paul Solari	Spark
Industry – CSL Behring	Karen Pynachian	CSL Behring
WFH WBDR Co-Chair	Alfonso Iorio	McMaster University, Hamilton, Canada

### WFH GTR Industry Consortium

Representation	Name	Affiliation
Co-chair, WFH Gene Therapy Registry Scientific Advisory Board	Barbara Konkle	University of Washington, Seattle, United States
Co-chair, WFH Gene Therapy Registry Scientific Advisory Board	Flora Peyvandi	Università degli Studi di Milano, Milan, Italy
VP Medical, WFH	Glenn Pierce	WFH
Industry	Jasmine Healy	Pfizer
Industry	Lisa Wilcox	Pfizer
Industry	Carole Paley	Biomarin
Industry	David Hinds	Biomarin
Industry	Paul Solari	Spark
Industry	Jonathan Schelfhout	Spark
Industry	Karen Pinachyan	CSL Behring
Industry	Blanca Salazar	CSL Behring

### WFH GTR Patient Advisory Group

Representation	Name	Affiliation/Country
Chair, WFH Gene Therapy Registry Steering Committee	Barbara Konkle	University of Washington, Seattle, United States
VP Medical, WFH	Glenn Pierce	WFH
Patient representative	Bradley Rayner	South Africa
Patient representative	Brendan Hayes	United States
Patient representative	Brian O'Mahony	Ireland
Patient representative	David Page	Canada
Patient representative	Laurence Woollard	United Kingdom
Patient representative	Mark Skinner	United States