

# GENE THERAPY FOR HEMOPHILIA

A curated repository of materials about Gene Therapy in the Shared Decision-Making context and scientific publications on Gene Therapy research for the Hemophilia community

This list of resources was compiled in consultation with Patient Advocacy Organizations. BioMarin is not responsible for the content of the linked resources and has not confirmed whether the resources listed here are complete, accurate, or up-to-date. This material is non-promotional and has as a sole objective to present scientific information regarding diseases and/or health. BioMarin makes no warranty or representations regarding the materials listed here. The content should not replace additional research and consultation with healthcare providers.

## BIOMARIN<sup>®</sup>

Co-developed in collaboration with the BioMarin Patient Council & Working Group members from the following Patient Advocacy Organizations:



Canadian Hemophilia Society  
Help Stop the Bleeding  
Société canadienne de l'hémophilie  
Arrêtons l'hémorragie



fedhemo  
Federación Española de Hemofilia



FUNDACIÓN  
DE LA HEMOFILIA  
Desde 1944



Federazione  
delle Associazioni  
EMOFILICI



Fondazione Paracelso  
nonostante l'emofilia.



Interessengemeinschaft  
Hämophiler e.V.



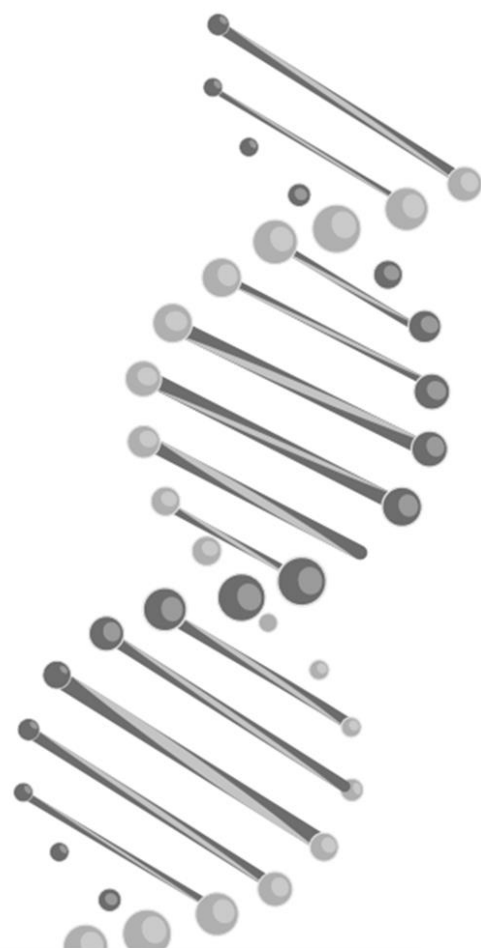
IRISH HAEMOPHILIA  
SOCIETY LTD  
Cumann Haemifile Na hEireann



NATIONAL  
BLEEDING DISORDERS  
FOUNDATION  
Formerly NHF



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



# WHAT WILL YOU FIND IN THIS DOCUMENT?

This document is a repository of **~50 identified** and **organized materials** about Gene Therapy literacy and shared decision-making, **complemented** by **~40 publications** on Gene Therapy research. The aim of this repository is to easily find educational materials regarding Gene Therapy in Hemophilia and support your learning journey. The list may be updated periodically to include new materials.

# HOW ARE MATERIALS CATEGORIZED?

Identified Gene Therapy literacy and shared decision-making materials and publications on Gene Therapy research are categorized within these key groups:



## Supranational/ Regional patient organizations

- World Federation of Hemophilia
- International Alliance of Patients' Organizations (IAPO)
- European Haemophilia Consortium

## Country-specific patient organizations



National Bleeding Disorders Foundation (formerly NHF)



IGH - Interessengemeinschaft Hämophiler e.V.



Haemophilia Foundation Australia



Canadian Hemophilia Society



Irish Haemophilia Society

## Medical/scientific organizations\* and others (e.g., Academia, consulting company)

- American Society of Gene & Cell Therapy (ASGCT)
- International Society on Thrombosis and Haemostasis (ISTH)
- European Association of Haemophilia and Allied Disorders (EAHAD)
- Ottawa Hospital Research Institute & University of Ottawa
- HAEMNET (The Journal of Haemophilia Practice)
- HAEMTRACK
- Pubmed



# IDENTIFIED GENE THERAPY & SHARED DECISION MAKING MATERIALS FROM SUPRANATIONAL PATIENT ORGANIZATIONS

	NAME	OBJECTIVE	YEAR	FORMAT	LINK
EUROPEAN HAEMOPHILIA CONSORTIUM	EHConversations: gene therapy series	To inform about gene therapy and hemophilia from both patient and expert points of view	2022		<a href="#">CLICK HERE</a>
	Gene therapy: A practical guide book	To help support and guide National Member organizations (NMOs) through the introduction of gene therapy as a therapeutic option in their countries	2022		<a href="#">CLICK HERE</a>
	EHCucate app	To provide information / education on rare bleeding disorders and novel therapies	2021		<a href="#">CLICK HERE</a>
IAPO/IFPMA	Cell and gene therapies toolkit	To provide science, technology, regulatory and safety information relevant to cell and gene therapy, together with tips on advocacy	2020		<a href="#">CLICK HERE</a>
WORLD FEDERATION OF HEMOPHILIA (WFH)	Introduction to gene therapy for hemophilia	To give short overview on gene therapy for hemophilia	2023		<a href="#">CLICK HERE</a>
	Clinical trials: Information on the eLearning platform	To educate the hemophilia community on the clinical trial process	2022		<a href="#">CLICK HERE</a>
	Clinical trials for hemophilia	To provide basic information about clinical trials for hemophilia	2022		<a href="#">CLICK HERE</a>
	Gene therapy: information on eLearning platform	To provide information on gene therapy	2022		<a href="#">CLICK HERE</a>
	Gene therapy for hemophilia clinical trials pipeline tool	To provide information on gene therapy clinical trials	2022		<a href="#">CLICK HERE</a>
	How new therapies get tested in clinical trials	To learn information for each phase of a clinical trial (e.g. study purpose, duration)	2022		<a href="#">CLICK HERE</a>
	What is gene therapy?	To provide basic patient information about gene therapy for hemophilia	2022		<a href="#">CLICK HERE</a>
	WFH Gene Therapy Registry - basics of hemophilia, gene therapy, and long-term data collection	To provide an introduction to the WFH Gene Therapy Registry	2022		<a href="#">CLICK HERE</a>
	WFH Gene Therapy Registry - user guide for people with hemophilia	To provide more information for people with hemophilia who wish to join the WFH Gene Therapy Registry	2022		<a href="#">CLICK HERE</a>
	WFH educational: Treatment choice in an era of change	To provide the patient and provider perceptions on treatment choices	2020		<a href="#">CLICK HERE</a>

## FORMAT TYPES



Mobile App



Handbook (PDF)



Interactive Tool



E-learning platform



Infographics



Video



# IDENTIFIED GENE THERAPY & SHARED DECISION MAKING MATERIALS FROM COUNTRY-SPECIFIC PATIENT ORGANIZATIONS

	NAME	OBJECTIVE	YEAR	FORMAT	LINK
IGH	Der Sommer & Gentherapie – beides zum Greifen nah! Aufzeichnung des 2. IGH-Gentherapie Web-seminar	To provide insights on the theory of gene therapy, the administration, follow-up and who could benefit from it ( <i>available in German only</i> )	2022		<a href="#">CLICK HERE</a>
HAEMOPHILIA FOUNDATION AUSTRALIA	Gene therapy for haemophilia	To answer the questions from Australian bleeding disorders community members about gene therapy and its relevance to them now and in the future	2019		<a href="#">CLICK HERE</a>
	Sharing knowledge makes us stronger	To discuss gene therapy studies and what it means for people with haemophilia	2018		<a href="#">CLICK HERE</a>
NATIONAL BLEEDING DISORDERS FOUNDATION (formerly NHF)	Gene therapy: eligibility – FAQs	To help understand who can undergo gene therapy	2022		<a href="#">CLICK HERE</a>
	Gene therapy: limitations, risks, and unknowns – FAQs	To provide answers to gene therapy: limitations, risks, and unknowns	2022		<a href="#">CLICK HERE</a>
	Gene therapy: the basics – FAQs	To provide answers to gene therapy basics	2022		<a href="#">CLICK HERE</a>
	Hemophilia treatment: now and the future	To provide expert education on all aspects of living with a bleeding disorder to chapters and HTC's (expired in 2021)	2021		<a href="#">CLICK HERE</a>
	Advancements in treatment for hemophilia: what you need to know	To provide an overview of new treatment options for hemophilia	2020		<a href="#">CLICK HERE</a>
	Gene therapy: A candid conversation	To have a balanced, patient-centric discussion on gene therapy from multiple perspectives	2020		<a href="#">CLICK HERE</a>
	Gene therapy: getting up to speed webinar	To present information on hemophilia and gene therapy	2020		<a href="#">CLICK HERE</a>

## FORMAT TYPES



Conference



Handbook (PDF)



Website



Webinar



Other



Video



# IDENTIFIED GENE THERAPY & SHARED DECISION MAKING MATERIALS FROM COUNTRY-SPECIFIC PATIENT ORGANIZATIONS

	NAME	OBJECTIVE	YEAR	FORMAT	LINK
NATIONAL BLEEDING DISORDERS FOUNDATION (formerly NHF)	Gene therapy: what's new & what's next webinar	To discuss the current status of gene therapies in the pipeline and other novel therapies	2020		<a href="#">CLICK HERE</a>
	What is gene therapy	To explain genes / gene therapy for hemophilia	2019		<a href="#">CLICK HERE</a>
	Gene therapy: questions for your provider	To help patients ask relevant hemophilia gene therapy questions to their provider	n/a		<a href="#">CLICK HERE</a>
	Gene therapy 101 for patients & families	To provide education on gene therapy and shared decision making	n/a		<a href="#">CLICK HERE</a>
	Gene therapy glossary of terms	To provide an overview of gene therapy terminology	n/a		<a href="#">CLICK HERE</a>
	Frequently asked questions on gene therapy	To answer to the most common questions asked about gene therapy for hemophilia	n/a		<a href="#">CLICK HERE</a>
CANADIAN HEMOPHILIA SOCIETY	All about hemophilia gene therapy: a guide for patients and caregivers	To provide the hemophilia community with basic information about gene therapy, its benefits and risks, what we know and don't know, and what questions we need to continue to try answering. The handbook is also available in French	2023		<a href="#">CLICK HERE</a>
IRISH HAEMOPHILIA SOCIETY	Novel treatments in haemophilia & other bleeding disorders: a periodic review 2023 – issue 1	To provide both up-to-date information to European Haemophilia Consortium National Member organizations (NMOs), and a general overview and understanding of a rapidly evolving landscape of medicinal product developments in rare bleeding disorders specific to haemophilia A and B; inhibitors in haemophilia, von Willebrand disease, and other rare bleeding disorders	2023		<a href="#">CLICK HERE</a>

## FORMAT TYPES



Conference



Handbook (PDF)



Website



Webinar



Q&amp;A



Other



Video



# IDENTIFIED GENE THERAPY & SHARED DECISION MAKING MATERIALS FROM MEDICAL/SCIENTIFIC ORGANIZATIONS & OTHERS

	NAME	OBJECTIVE	YEAR	FORMAT	LINK	
Medical/scientific organizations	EUROPEAN ASSOCIATION OF HAEMOPHILIA AND ALLIED DISORDERS (EAHAD)	16 <sup>th</sup> annual congress of the European association for hemophilia and allied disorders	2023		<a href="#">CLICK HERE</a>	
	AMERICAN SOCIETY OF GENE THERAPY & CELL THERAPY (ASGCT)	Hemophilia	2022		<a href="#">CLICK HERE</a>	
	INTERNATIONAL SOCIETY ON THROMBOSIS AND HAEMOSTASIS (ISTH)	Gene therapy for hemophilia A and B compared to available treatments	Discuss up-to-date information on the current and emerging approaches for treating hemophilia, including the various approaches of gene therapy. Discuss the pros and cons of gene therapy with patients and caregivers, including available resources for further education (expired in Dec. 2022)	2021		<a href="#">CLICK HERE</a>
Consulting company	PUBMED	Hemophilia gene therapy: your questions answered	2022		<a href="#">CLICK HERE</a>	
	HAEMNET (THE JOURNAL OF HAEMOPHILIA PRACTICE)	The journey of gene therapy in hemophilia – putting the patient at the centre of the hub and spoke model	To discuss the patient journey for gene therapy in hemophilia from a patient viewpoint	2023		<a href="#">CLICK HERE</a>
		Personalising hemophilia management with shared decision making	To advance the concept of shared decision making (SDM) between the hemophilia patient and their healthcare provider (HCP)	2021		<a href="#">CLICK HERE</a>
	HAEMTRACK	Haemtrack app	To enable patients to record all therapies as they occur, & allows clinicians to see up-to-date therapy information to help monitor, optimise & improve patient care	n/a		<a href="#">CLICK HERE</a>
Academia	OTTAWA HOSP. RESEARCH INSTITUTE & UNIVERSITY OF OTTAWA	Ottawa personal decision guide for people making health or social decisions	To help identify personal needs, plan the next steps, track progress, and communicate own views to others involved in the decision. The learnt skills will also help them make other decisions in the future	2015		<a href="#">CLICK HERE</a>

FORMAT TYPES



Video (and content)



Video



Congress



Publication



Podcast



Mobile App



Toolkit



Q&amp;A

# Contact information

Contact information from organizations that have co-authored this resource and/or issued any of the materials featured

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Global Medical Information

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Website: <https://www.hemophilia.ca/>

## European Haemophilia Consortium

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## Federación Española De Hemofilia

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## Fundación de la Hemofilia

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## International Alliance of Patients' Organizations

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## Irish Haemophilia Society

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## National Bleeding Disorders Foundation (formerly NHF)

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Toll-free Number: 888 463 6643

✉ [info@hemophilia.org](mailto:info@hemophilia.org)

Website: <https://www.hemophilia.org/>

## World Federation of Hemophilia

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