

Gene Therapy for Hemophilia Clearing House: a single repository of resources for hemophilia

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INTRODUCTION

- Adeno-associated virus (AAV)-based gene therapies (GTs) are innovative treatments for hemophilia that aim to increase endogenous production of important clotting factors and attenuate bleeding episodes with a single treatment session¹
- Individuals with hemophilia considering AAV-GT should have access to information covering all aspects of the patient journey; however, there is no single repository that provides updated patient literacy resources on GT for hemophilia
 - Patient organizations (POs) develop educational materials; however, oftentimes topics are duplicated or missed across materials
- To address this, members of the Global Hemophilia Patient Council (GHPC; **Figure 1**) collaborated to develop the 'Clearing House of Gene Therapy for Hemophilia,' a curated repository of educational resources created to guide and enhance hemophilia GT education and global awareness

Figure 1. Hemophilia POs of the GHPC who participated in the 'Clearing House of Gene Therapy for Hemophilia' collaboration

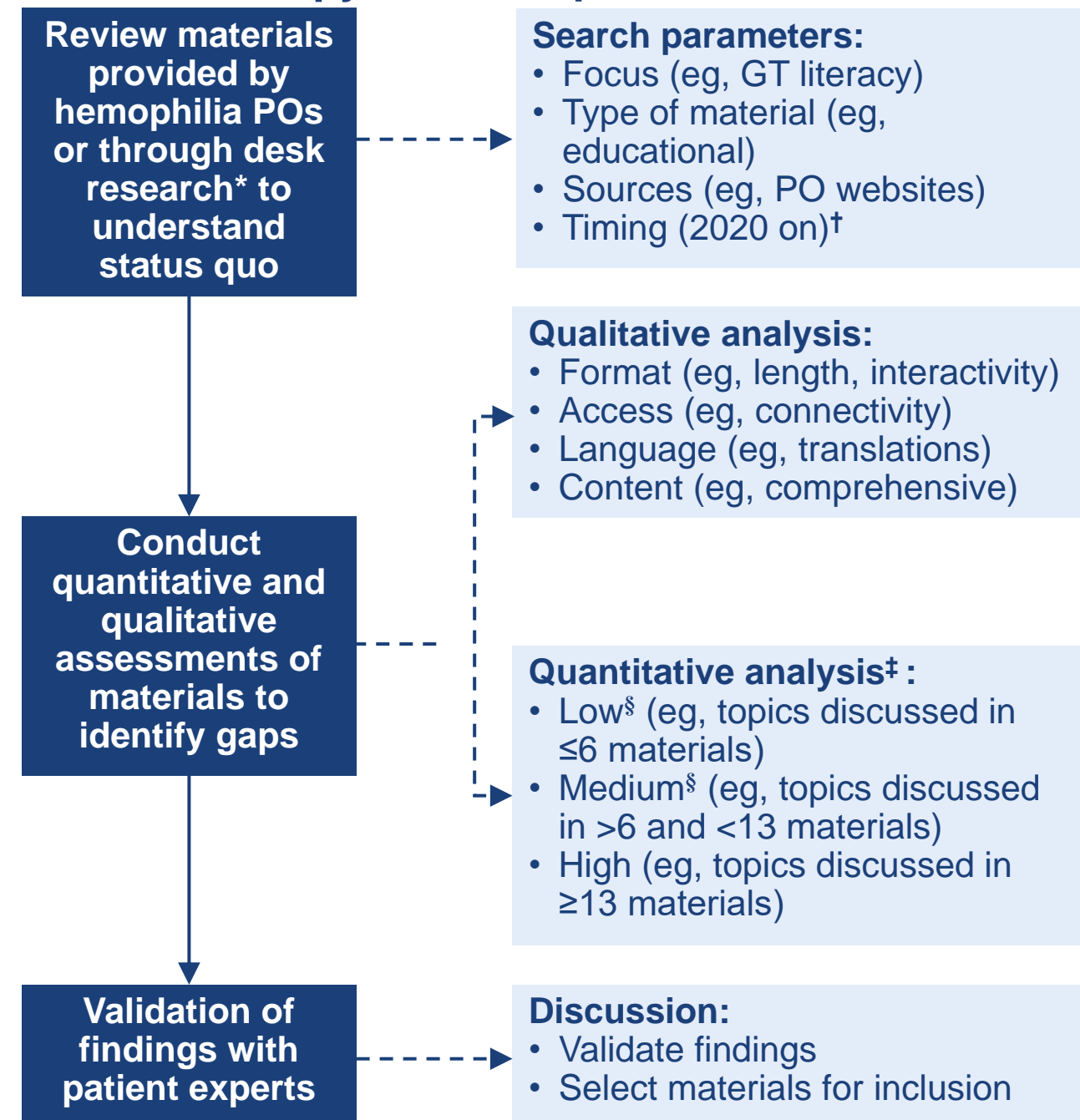


The Association Française des Hémophiles also participated in the development of the Clearing House. GHPC, Global Hemophilia Patient Council; PO, patient organization.

METHODS

- Educational materials pertaining to hemophilia GT were sourced from participating hemophilia POs and desk research (industry-created materials not considered)
- Sourced materials were quantitatively and qualitatively assessed (**Figure 2**)
 - Quantitative assessments of topics discussed in the materials were measured on a scale of low (included in ≤6 materials), medium (>6 and <13 materials), and high (≥13 materials) to identify topic gaps
 - The quality of the material was based on 4 tenets: format, accessibility, language, and content
- High-quality resources that presented topics identified as gaps were discussed by the GHPC, which validated and selected materials for inclusion in the repository

Figure 2. Approach to create the 'Clearing House of Gene Therapy for Hemophilia'



*Industry-created materials were not considered for inclusion. †Except for 4 materials. Quantitative analyses aimed to map available materials against the GT journey to assess the scope and volume of materials. *Topics characterized as low and medium were identified as gaps. GT, gene therapy; PO, patient organization.

ACKNOWLEDGEMENTS

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1. Nathwan AC. *Hematology Am Soc Hematol Educ Program*. 2022;2022(1):569-78.

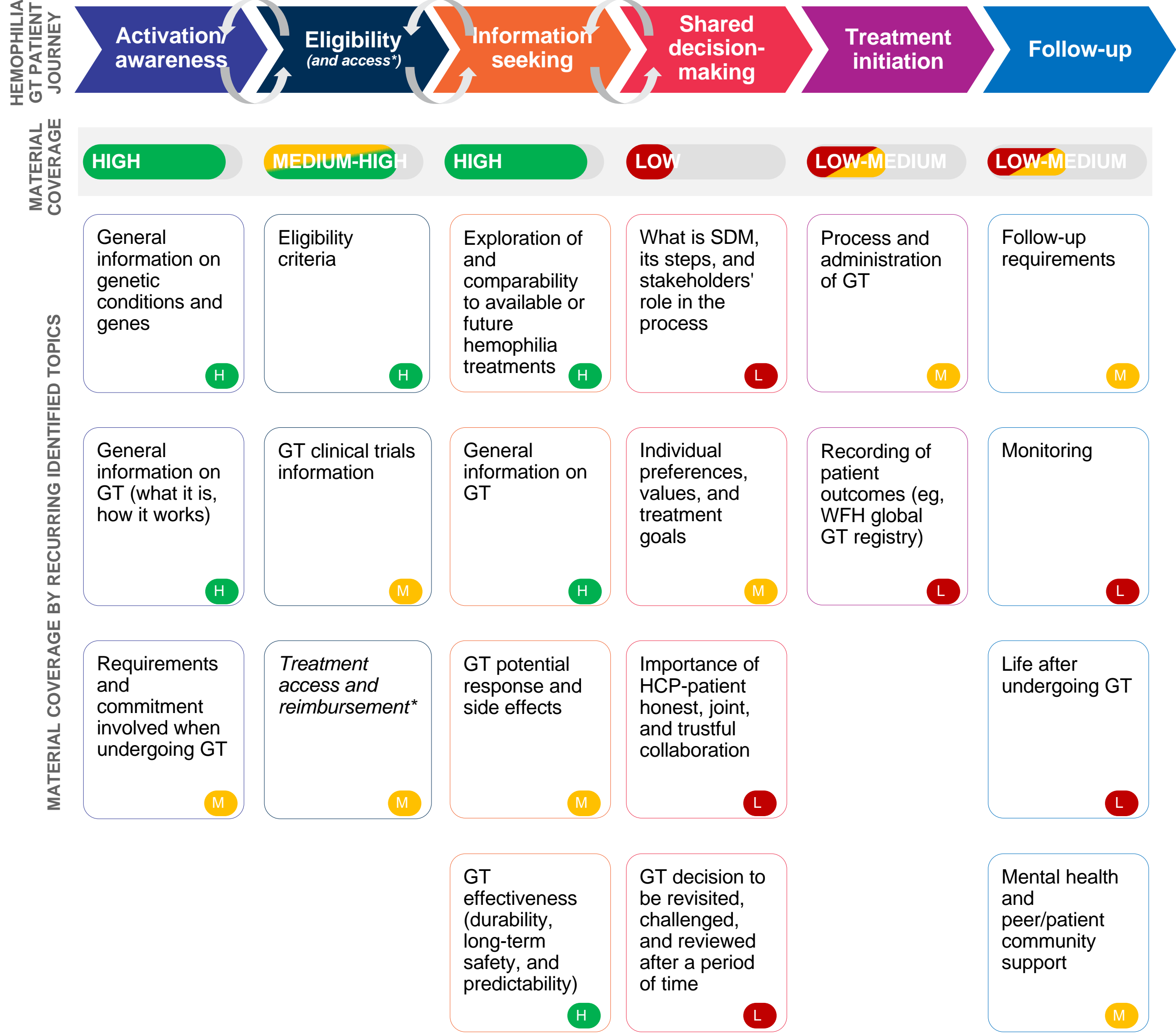
CONFLICTS OF INTEREST

Brian O'Mahony serves as the CEO of the Irish Haemophilia Society. Tobias Becker serves as a board member of IGH - Interessengemeinschaft Hämophiler e.V. Carol Betsch is an employee and the CEO of the Association française des hémophiles. Andrea Buzzi serves as a board member of Fondazione Paracelso. Donna Coffin serves as a board member of the World Federation of Hemophilia. Enrico Ferri Grazzi serves as a board member of the Federazione delle Associazioni Emofili. Daniel-Anibal García Diego serves as a board member of Fedhemo - Federación Española de Hemofilia. Nicolas Giraud serves as a board member of the Association française des hémophiles. Antonio Gomez Cavallini serves as a board member of the Fundación de la Hemofilia. Dor Goshen serves as a board member for the Israeli Hemophilia Association. Brendan Hayes serves as an employee of the National Bleeding Disorders Foundation. David Page serves as a consultant on coagulation product safety and supply issues for Canadian Hemophilia Society. Dawn Rotellini serves as an officer of the National Bleeding Disorders Foundation and a board member of the World Federation of Hemophilia. Clive Smith serves as a board member of The Haemophilia Society. Jonathan Liu is an employee and shareholder of BioMarin Pharmaceutical Inc. Sanja Njelic is an employee and shareholder of BioMarin Pharmaceutical Inc.

RESULTS

- From the materials quantitatively and qualitatively assessed, GT literacy, shared decision-making, and outcomes following GT were identified as gaps and became the primary subject matter of the repository (**Figures 3 & 4**)

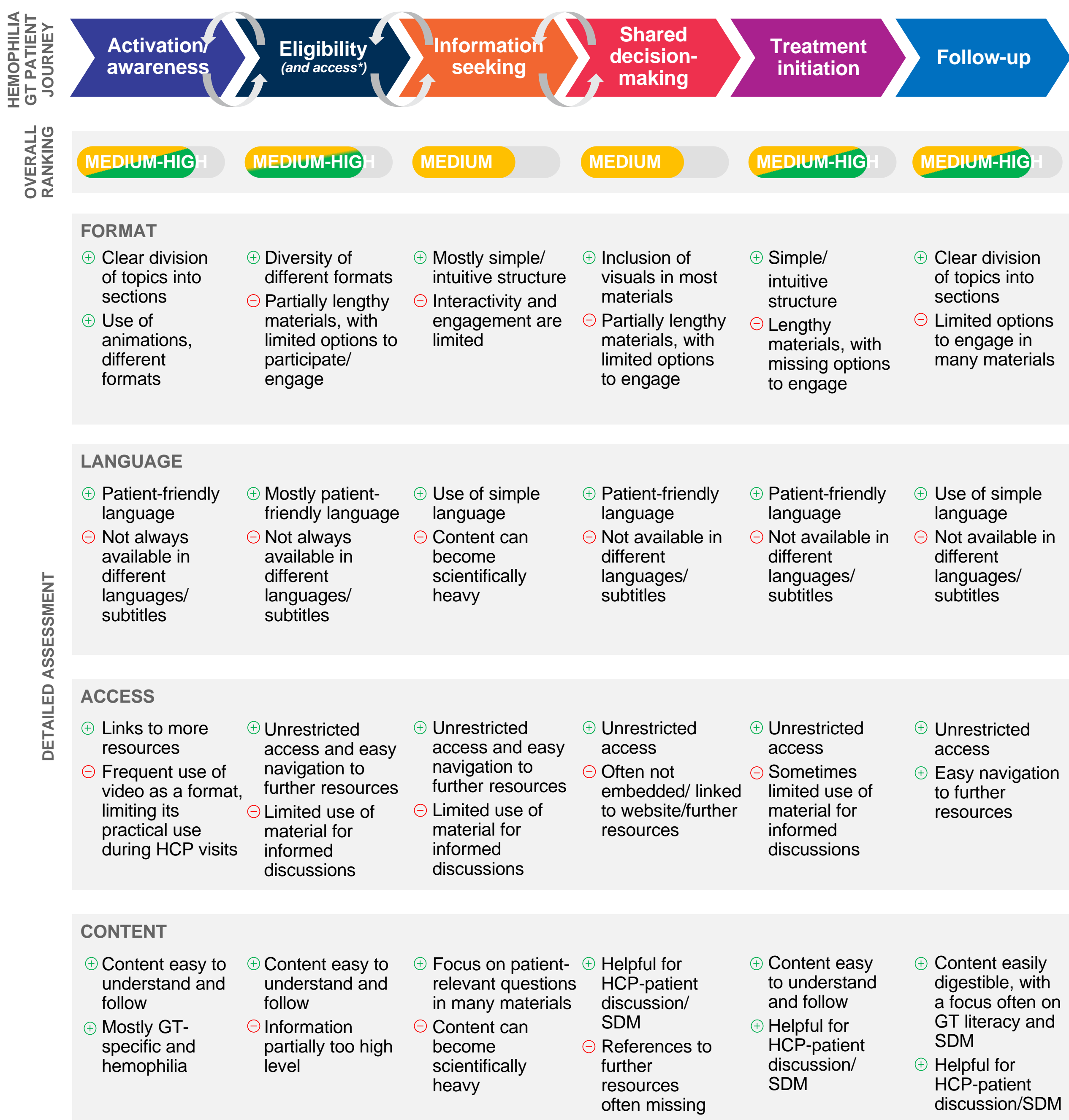
Figure 3. Quantitative analysis of identified materials across hemophilia GT patient journey



○ = iterative process

*Access and reimbursement of GT will be country-dependent and should be considered separately. GT, gene therapy; L, low (≤6 materials); H, high (≥13 materials); HCP, health care provider; M, medium (>6 and <13 materials); SDM, shared decision-making; WFH, World Federation of Hemophilia.

Figure 4. Aggregated output of qualitative assessment



○ = iterative process

Output demonstrates an aggregated version of the qualitative assessment and is thus not representative for each material individually. *Access of GT will be country-dependent and should be considered separately. GT, gene therapy; HCP, health care provider; SDM, shared decision-making.

CONCLUSIONS

- This unique repository will be updated regularly, as it aims to serve as an up-to-date educational resource to support the patient and their physician's shared learning journey regarding hemophilia gene therapy
- To improve accessibility, the 'Clearing House of Gene Therapy for Hemophilia' will soon be available in different languages
- The development of the Clearing House promoted sharing of best practices, which augmented the repository as a whole; continued collaboration between POs will help eliminate duplication and improve quality of future materials

- Content within the 'Clearing House of Gene Therapy for Hemophilia' gives a holistic overview of what the patient journey will look like, including topics such as gene therapy literacy, shared decision-making, and outcomes following AAV-GT
- In total, the Clearing House contains references and links to ~50 organized materials that are provided in multiple formats that use plain language and provide flexible ways to learn about gene therapy (**Tables 1–3**)

Table 1. Identified GT and SDM materials from supranational patient organizations

	NAME	OBJECTIVE	YEAR	FORMAT
European Haemophilia Consortium	EHCConversations: GT series	To inform about GT and hemophilia from both patient and expert points of view	2022	Video
	GT: a practical guide book	To help support and guide National Member organizations through the introduction of GT and therapeutic options in their countries	2022	Handbook (PDF)
	EHCucate app	To provide information/education on rare bleeding disorders and novel therapies	2021	Mobile app
IAPO/IFPMA	Cell and GTs toolkit	To provide science, technology, regulatory, and safety information relevant to cell and GT, together with tips on advocacy	2020	Handbook (PDF)
	Introduction to GT for hemophilia	To give a short overview on GT for hemophilia	2023	Video
	Clinical trials: information on the eLearning platform	To educate the hemophilia community on the clinical trial process	2022	E-learning platform
World Federation of Hemophilia	Clinical trials for hemophilia	To provide basic information about clinical trials for hemophilia	2022	Handbook (PDF)
	GT: information on eLearning platform	To provide information on GT	2022	E-learning platform
	GT for hemophilia clinical trials pipeline tool	To provide information on GT clinical trials	2022	Interactive tool
	How new therapies get tested in clinical trials	To learn information for each phase of a clinical trial (eg. study purpose, duration)	2022	Infographics
	What is GT?	To provide basic patient information about GT for hemophilia	2022	Handbook (PDF)
	WFH GT Registry-basics of hemophilia, gene therapy, and long-term data collection	To provide an introduction to the WFH GT Registry	2022	Handbook (PDF)
	WFH GT Registry-user guide for people with hemophilia	To provide more information for people with hemophilia who wish to join the WFH GT Registry	2022	Handbook (PDF)
	WFH educational: treatment choice in an era of change	To provide the patient and provider perceptions on treatment choices	2020	Video

EHC, European Haemophilia Consortium; E-learning, electronic learning; GT, gene therapy; IAPO/IFPMA, International Alliance of Patient Organizations/International Association of Pharmaceutical Manufacturers and Associations; SDM, shared decision-making; WFH, World Federation of Hemophilia.

Table 2. Identified GT and SDM materials from country-specific patient organizations

	NAME	OBJECTIVE	YEAR	FORMAT
IGH	Der Sommer und Gentherapie – beides zum Gelingen näh! Aufzeichnung des 2. IGH-Gentherapie web-seminar	To provide insights on the theory of GT, the administration, follow-up, and who could benefit from it (available in German only)	2022	Video
Haemophilia Foundation Australia	GT for haemophilia	To answer the questions from Australian bleeding disorders community members about GT and the relevance to them now and in the future	2019	Handbook (PDF)
	Sharing knowledge makes us stronger	To discuss GT studies and what GT means for people with haemophilia	2018	Handbook (PDF)
	GT: eligibility – FAQs	To help understand who can undergo GT	2022	Video
	GT: limitations, risks, and unknowns – FAQs	To provide answers to GT: limitations, risks, and unknowns	2022	Video
	GT: the basics – FAQs	To provide answers to GT basics	2022	Video
National Bleeding Disorders Foundation (formerly NHF)	Hemophilia treatment: now and the future	To provide expert education on all aspects of living with a bleeding disorder to chapters and HTCs (expired in 2021)	2021	Conference
	Advancements in treatment for hemophilia: what you need to know	To provide an overview of new treatment options for hemophilia	2020	Video
	GT: a candid conversation	To have a balanced, patient-centric discussion on GT from multiple perspectives	2020	Webinar
	GT: getting up to speed webinar	To present information on hemophilia and GT	2020	Webinar
	GT: what's new & what's next webinar	To discuss the current status of GTs in the pipeline and other novel therapies	2020	Webinar
	What is GT	To explain genes/GT for hemophilia	2019	Video
	GT: questions for your provider	To help patients ask relevant hemophilia GT questions to their provider	n/a	Handbook (PDF)
	GT 101 for patients & families	To provide education on GT and SDM	n/a	Video
	GT glossary of terms	To provide an overview of GT terminology	n/a	Other
	FAQs on GT	To answer to the most common questions asked about GT for hemophilia	n/a	Q&A
Canadian Hemophilia Society	Gene Therapy Education Program	To provide the hemophilia community with basic information about GT, its benefits and risks, what we know and don't know, and what questions we still need answered	2024	Booklet, podcasts, webcasts
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 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	Handbook (PDF)

FAQs, frequently asked questions; GT, gene therapy; HTC, Hemophilia Treatment Centers; IGH, Interessengemeinschaft Hämophiler e.V.; n/a, not available; NHF, National Hemophilia Foundation; SDM, shared decision-making.

Table 3. Identified GT and SDM materials from medical/scientific organizations and others

MEDICAL/SCIENTIFIC ORGANIZATIONS	NAME	OBJECTIVE	YEAR	FORMAT
European Association of Haemophilia and Allied Disorders	17th Annual Congress of the European Association for Haemophilia and Allied Disorders	To promote education, support research, and improve the clinical care and lives of patients with inherited or acquired bleeding disorders (17th Annual Congress of the European Association for Haemophilia and Allied Disorders 2024. <i>Haemophilia</i> . 2024;30:1-201)	2023	Congress
	American Society of Gene & Cell Therapy (ASGCT)	Hemophilia	To provide basic information about hemophilia and GT	2022
International Society on Thrombosis and Haemostasis (ISTH)	GT for hemophilia A and B compared to available treatments	Discuss up-to-date information in the current and emerging approaches for treating hemophilia, including the various approaches of GT, discuss the pros and cons of GT with patients and caregivers, including available resources for further education (expired in Dec 2022)	2021	Podcast
PubMed	Hemophilia GT: your questions answered	To provide answers to questions across the 5 stages of the patient decision-making journey (as part of the publication Wang M, et al. <i>Patient Prefer Adherence</i> . 2022;16:1439-47)	2022	Q&A
CONSULTING COMPANY				
Haemnet (The Journal of Haemophilia Practice)	The journey of GT in hemophilia—putting the patient at the center of the hub and spoke model	To discuss the patient journey for GT in hemophilia from a patient viewpoint	2023	Publication
	Personalizing hemophilia management with SDM	To advance the concept of SDM between the patient with hemophilia and their HCP	2021	Publication
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, optimize, and improve patient care	n/a	Mobile app
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 learned skills will also help patients make other decisions in the future	2015	Toolkit

GT, gene therapy; HCP, health care provider; Hosp., hospital; n/a, not available; Q&A, question and answer; SDM, shared decision-making.

Scan to view the Clearing House online

