EHC

european haemophilia consortium

Advocating for people with haemophilia and congenital bleeding disorders

ANNUAL REPORT
2022

INTRODUCTION

MESSAGE FROM THE STEERING COMMITTEE

In 2022, we saw a promising start to the year, with the return of in-person meetings and events. Notable gatherings included the youth workshop, women's conference in Basel, leadership conference in Brussels, and the successful EHC conference in Copenhagen. These gatherings provided an opportunity for our community to reconnect, exchange information, and discuss treatment and events.

Marseille hosted the New Technologies workshop. The EHC reaffirmed its commitment to inclusivity by adding "EHC, advocating for people with haemophilia and congenital bleeding disorders" to our logo. We also reevaluated the Inhibitor Working Group, leading to the establishment of the European Rare and Inhibitor Working Group (ERIN), culminating in a joint encounter of patients with Rare Bleeding Disorders and Inhibitor patients at Barretstown.

These encounters ignited renewed enthusiasm within our organisation. Committees increased their efforts, holding online meetings, happy hours, and community calls. The VWD committee made substantial progress with the implementation of guidelines and improved organisation, including the search for ambassadors.

The women's conference highlighted the ongoing need for recognition and adequate treatment for women facing difficulties related to bleeding disorders. Our Think Tank delved into workstreams, resulting in the publication of the first document, "A report from the 1st workshop of the EHC Think Tank Workstream on Registries."

In response to evolving community needs, EHC addressed the ageing of patients with bleeding disorders through a survey presented at the EHC conference. We also initiated programs to support the health of our member organisations and explored opportunities for internships within our organisation. The EHC also increased its involvement towards the mild bleeding disorders and as of December 2022, partnered with the EHA for the development of guidelines for this very specific population.

Keeping a close eye on research and therapeutic advancements, EHC recognized the significance of gene therapy. To facilitate discussions between care providers and patients, we authored a comprehensive "Gene Therapy Guidebook" to address the physical and mental aspects of gene therapy decision-making.

Despite the considerable resources required in 2022, both in terms of human effort and finances, EHC experienced encouraging moments. Thanks to the dedication of volunteers, committee members, staff, NMOs, MAG and MASAG members, and sponsors, we were able to rekindle our mission post-Covid. We extend our gratitude to all who have contributed to our journey, providing the foundation upon which EHC continues to evolve towards future horizons.

EHC

european haemophilia consortium

Advocating for people with haemophilia and congenital bleeding disorders

Together towards better inclusion!

At the EHC Leadership Conference 2022, the new logo with a tagline that better reflects EHC mission - representing 48 national patient organisations for people with all rare bleeding disorders - was revealed. The EHC, "Advocating for people with haemophilia and congenital bleeding disorders."

EHC IN ESSENCE

The European Haemophilia Consortium (EHC) is an international non-profit organisation representing 48 national patients' organisations for people with rare bleeding disorders from 27 Member States of the European Union (EU) and most Member States of the Council of Europe.

The EHC represents more than 90,000 people diagnosed with a rare bleeding conditions such as haemophilia, von Willebrand Disease (VWD) and other rare bleeding disorders across Europe. However, experts estimate that many more live with an undiagnosed rare bleeding disorder.

The EHC actively supports its National Member Organisations (NMOs) at national and European levels and also helps NMOs to engage with each other, with the objectives of:

- Improving the quality of life of people living with rare bleeding disorders;
- Improving diagnostic and treatment facilities;
- Ensuring the adequate supply of – and access to – safe therapies;
- Promoting patients' rights and raising ethical issues;
- Following and influencing developments in European health policy;
- Understanding the status of treatment and care in member countries through regular surveys;
- Stimulating research in all fields related to haemophilia and related rare bleeding disorders.

The EHC draws on the knowledge of patients, healthcare professionals, the scientific community, the European institutions and the pharmaceutical industry to share expertise within Europe. The EHC also collaborates closely with other European patient organisations to ensure a strong collective voice for people with rare bleeding disorders.

EHC through the eyes of the community
(Leadership Conference 2022)

MEMBERSHIP

In 2022, the organisations here below were part of the EHC membership:

Albania	Shoqatës Shqiptare të Hemofilikëve
Armenia	Armenian Association of Hemophiliacs
Austria	Österreichische Hämophilie Gesellschaft (ÖHG)
Azerbaijan	Hemofiliyalı Xəstələrin Respublika Assosiasiyası
Belarus	Белорусская Ассоциация Больных Гемофилией
Belgium	Association de l'Hémophilie - Hemofilievereniging (AHVH)
Bosnia and Herzergovina	Udruženje Hemofiličara Bosne i Hercegovine
Bulgaria	Българска Асоциация по Хемофилия
Croatia	Društvo hemofiličara Hrvatske
Cyprus	Παγκύπρια Οργάνωση Αιμορροφιλικών (Π.Ο.Α.)
Czech Republic	Český svaz hemofiliků
Denmark	Bløderforening
Estonia	Eesti Hemofiliauhing
Finland	Suomen Hemofiliayhdistys (SHY)
France	Association Française des Hémophiles (AFH)
Georgia	Georgian Association of Hemophilia and Donorship
Germany	Deutsche Hämophilie Gesellschaft
Greece	Σύλλογος Προστασίας Ελλήνων Αιμορροφιλικών (ΣΠΕΑ)
Hungary	Magyar Hemofília Egyesület
Iceland	Blæðarafélag Íslands
Ireland	Irish Haemophilia Society
Israel	היליפומה ילוחל התומע – הלע
Italy	Federazione delle Associazioni Emofilici (Fedemo)
Kyrgyz Republic	Kyrgyz Haemophilia Society "Community of handicapped – haemophiliacs"
Latvia	Latvijas Hemofilijas Biedrība
Lithuania	Lietuvos Hemofiljos Associja
Luxembourg	Association Luxembourgeoise des Hémophiles
Malta	Maltese Bleeding Disorders Society
North Macedonia	Hemolog
Moldova	Ассоциации Гемофилии Молдовы
Montenegro	Montenegrin Association for Hemophilia
Netherlands	Nederlandse Vereniging van Hemofilie-Patiënten (NVHP)
Norway	Foreningen for blødere i Norge (FBIN)
Poland	Polskie Stowarzyszenie Chorych na Hemofilię
Portugal	Associação Portuguesa de Hemofilia e de outras Coagulopatias Congénitas (APH)
Romania	Asociația Română de Hemofilie (ARH)
Russia	Всероссийское общество гемофилии
Serbia	Udruženje hemofiličara Srbije
Slovak Republic	Slovenské hemofilické združenie
Slovenia	Društvo hemofilikov Slovenije
Spain	Federación Española de Hemofilia (FEDHEMO)
Sweden	Förbundet Blödarsjuka i Sverige
Switzerland	Schweizerische Hämophilie-Gesellschaft
Tajikistan	gemofily_tj@mail.ru
Turkey	Türkiye Hemofili Derneği
Ukraine	Всеукраїнське товариство Гемофілії
United Kingdom	The Haemophilia Society
Uzbekistan	Узбекское общество гемофилии

STEERING COMMITTEE

In March 2022, the EHC Steering Committee and staff met face-to-face in Genval, Belgium for a strategic retreat. During this two-day meeting the EHC strategic direction was revisited and different working areas discussed in order to find the best ways to serve the community and to navigate the post-COVID-19 reality. The retreat also provided valuable moments of team building among the Steering Committee members and staff.

During the EHC Conference 2022 in Copenhagen Minette van der Ven ended her term as Vice-President Finance and Manon Degenaar-Dujardin was elected for this position. The vacancy in the EHC Steering Committee was filled by co-opting Evelyn Grimberg from the Dutch NMO in November.

The EHC would like to take the opportunity to warmly thank Minette for the time and energy invested into working on EHC finances!

The EHC 2022 Steering Committee members were:

Declan Noone
President, Irish NMO

Minette van der Ven
Vice-President Finance,
Dutch NMO (until October)

Miguel Crato
Steering Committee Member,
Portuguese NMO

Thomas Sannié
Steering Committee Member,
French NMO

Manon Degenaar-Dujardin
Steering Committee Member,
Dutch NMO

Stefan Radovanovic
Steering Committee Member,
Serbian NMO

Jo Eerens
Steering Committee Member,
Belgian NMO

Diana Lighezan
Steering Committee Member,
Romanian NMO

Evelyn Grimberg
Steering Committee Member,
Dutch NMO
(from November)

Amanda Bok
Ex-Officio Member, EHC CEO

Medical Advisory Group (MAG)

The EHC MAG is composed of experts who assist the EHC with high-level medical and scientific support and guidance and provide strategic input to the EHC SC and staff. The MAG is consulted on ad hoc matters and is a valued source of expertise and know-how.

In 2022, the MAG members were:

- **Michael Makris** (Chairperson) | United Kingdom
- **Flora Peyvandi** | Italy
- **Jan Blatný** | Czechia
- **Johannes Oldenburg** | Germany

Medical and Scientific Advisory Group (MASAG)

The MASAG forms a comprehensive team of medical and scientific experts, providing the EHC with advice and guidance on specific areas of EHC activities with a multidisciplinary approach. The MASAG is a strong network of professionals ensuring strategic input into our programmes and activities, and assisting the EHC with hands-on and personalised support during specific events and activities. The MASAG is also consulted on some legislative matters to provide the EHC with reliable and relevant expertise on key areas of work.

Members of the MASAG were:

- **Rezan Abdul-Kadir** | United Kingdom
- **Massimo Colombo** | Italy
- **Piet De Kleijn** | The Netherlands
- **Roseline d'Oiron** | France
- **Alison Dougall** | Ireland
- **Carmen Escuriola-Ettinghausen** | Germany
- **Albert Farruggia** | Australia
- **Dan Hart** | United Kingdom
- **Anneliese Hilger** | Germany
- **Vince Jenkins** | United Kingdom
- **Radoslaw Kaczmarek** | USA
- **Evelien Mauser-Bunschoten** | The Netherlands
- **Giuseppe Mazza** | United Kingdom
- **Beatrice Nolan** | Ireland
- **Cristina Novembrino** | Italy
- **Jamie O'Hara** | United Kingdom
- **Brian O'Mahony** | Ireland
- **Debra Pollard** | United Kingdom
- **Frits Rosendaal** | The Netherlands
- **Glenda Silvester** | United Kingdom
- **Luigi Solimeno** | Italy
- **Thierry Vanden Driessche** | Belgium

Amanda Bok
CEO

Saskia Pfeyffer
Office Assistant

Fiona Brennan
Community Programmes Officer

Daria Mironova
Communications and Public Policy Officer

Zita Gacser
Think Tank Officer

Florian Francois
Office Assistant

Naja Skouw-Rasmussen
Think Tank Officer

Kristine Jansone
Community Programmes Officer

Laura Savini
Communications and Public Policy Officer

Rumana Molla
Executive Assistant to CEO and President

Jeanette Fava
Operations Director

EHC STAFF & ORGANISATION

In the beginning of 2022, the day-to-day work of the EHC was carried out by a team of dedicated professionals composed of Amanda Bok, Fiona Brennan, Kristine Jansone, Daria Mironova, Rumana Molla, Saskia Pfeyffer, Laura Savini, and Naja Skouw-Rasmussen.

In February, the EHC team was enriched by Zita Gacser, who took up the role of the second Think Tank Officer. In May, another team change took place and Florian Francois joined the EHC for a temporary role of Office Assistant to brighten the days of the EHC staff with sparkling personality and great support. In June, Jeanette Fava joined the EHC team as Operations Director to support the financial and administrative work of the EHC.

To support EHC team members through the changes in the team and return to the post-COVID-19 reality, an organisational coach, Svetoslava Stoyanova was engaged. The EHC is deeply grateful to Svetoslava for all the discussions, insights, and support!

EHC IN ACTION

The EHC's key role consists in shaping the quality of life of all people with congenital bleeding disorders in Europe, through partnership and advocacy: in substance, in form, and into the future. The EHC's day-to-day work is guided by its Strategic Objectives, developed by the EHC Steering Committee, Medical Advisory Group, Medical and Scientific Advisory Group and EHC staff, based on the needs of the bleeding disorders community for a period of five years.

In 2022, the Strategic Objectives of the EHC were:

1

ADVOCACY AND AWARENESS

To advocate and partner effectively towards improving quality of life for all people with congenital bleeding disorders in Europe.

- Rare disease day 2022
- World Haemophilia Week 2022
- EHC multimedia exhibition "Open the Door to Another Reality!"
- EHC Conference 2022
- EHC Think Tank
- European principles of care for physiotherapy provision for persons with inherited bleeding disorders: Perspectives of physiotherapists and patients
- Partnerships

2

EDUCATION, ENGAGEMENT AND DEVELOPMENT

To ensure state-of-the-art knowledge and relevant skills for National Member Organisation (NMO) representatives to help them achieve better treatment and comprehensive care nationally.

- EHC Educational App EHCucate
- New Technologies Workshop
- EHC Workshop on Health Economics
- EHC Novel Treatment Products Newsletter
- New Products Working Group
- #thisway campaign

3

COMMUNITY EMPOWERMENT AND SUPPORT

To empower NMOs and support their future development.

- EHC Youth Programme
- EHC Leadership Conference
- Cohort-specific Programmes
- Women with Bleeding Disorders
- Von Willebrand Disease
- European Rare and Inhibitor Network (ERIN)
- Community Advisory Boards
- NMO support & Community
- Activity Grants and Physical Activity Grants
- Emergency Displacement Fund

4

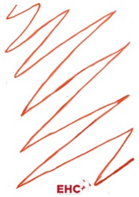
SUSTAINABILITY

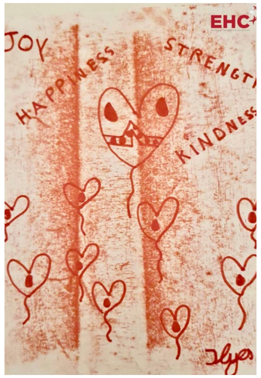
To ensure proof of EHC's organisational health and secure its future development.

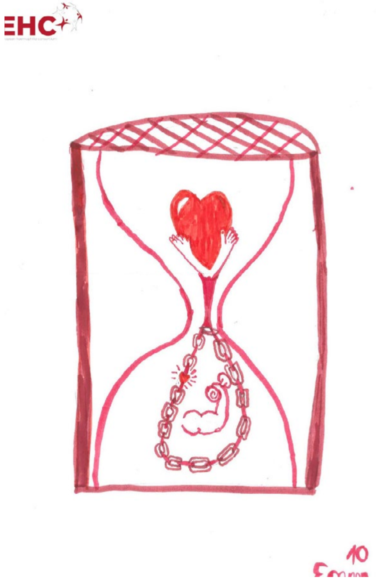
- EHC Organisational Health Programme
- EHC Income and Expenditure
- Independent Auditor's Report
- Balance Sheet

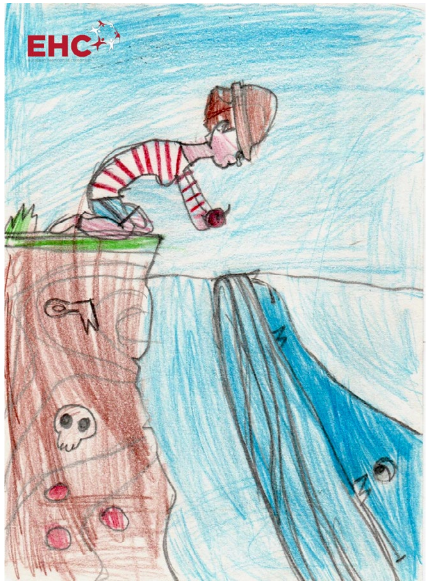
Rare Disease Day 2022

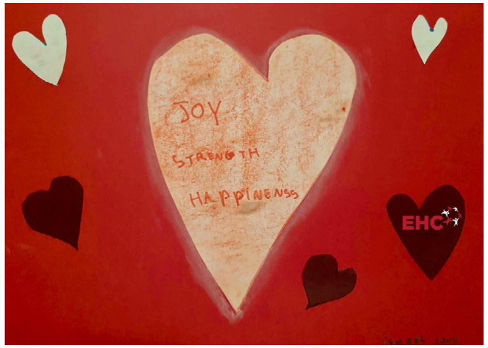
In order to raise awareness of bleeding disorders as being part of a broader rare disease community, and to highlight the incredible courage and strength it takes to live with a rare bleeding disorder, the EHC asked the younger generation of the community to support others through drawings Several beautiful artworks were received on the theme of strength and kindness.









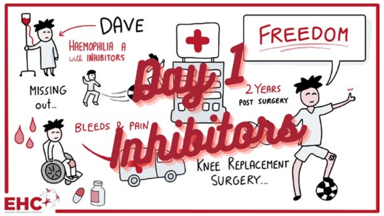


World Haemophilia Week 2022

Each year, the haemophilia and rare bleeding disorders community worldwide marks World Haemophilia Day on 17 April. This commemoration is an opportunity to raise awareness about the needs of those affected by rare bleeding disorders.

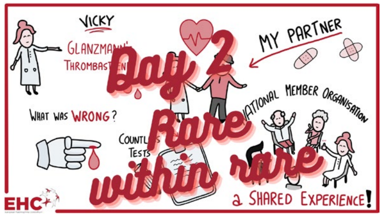
In 2022, the theme of the EHC World Haemophilia Week (WHW) was Access for All and it focused on groups within the bleeding disorders community that have traditionally received less attention and are often struggling to get recognition and adequate access to treatment and care. These groups are people with inhibitors, women with bleeding disorders, people with extremely rare bleeding disorders, people with von Willebrand Disease and people with mild and moderate bleeding disorders. Every day from 17 to 22 April 2022, the EHC released an educational video tackling one of the aforementioned topics, as well as providing resources on the EHC website for further information and education. The EHC is deeply grateful to all the volunteers and healthcare professionals that contributed to the content and preparation of the World Haemophilia Week.

April 18



The focus was on haemophilia with **inhibitors**, featuring themes, such as isolation and importance of the community, surgery and considerations around it for people with inhibitors, as well as the role of physiotherapy and being physically active.

April 19



The themes relevant to people living with **extremely rare bleeding disorders** were highlighted - seeking the information about the diagnosis and care; bleeding complications in the mouth and dental care; and impact of the bleeding disorder on the body image and how to deal with it.

April 20

The day that focused **mild and moderate (non-severe) bleeding disorders**, the need to change the narrative around bleed assessment, redefining what is "normal" in terms of bleeds, was discussed; as well as advice for those patients on how to best navigate the healthcare system and the approaches for better engagement of the patients with milder bleeding disorders into the patient community were offered.

April 21

Women with bleeding disorders were in the spotlight. The importance of early and accurate diagnosis was underlined; the advice and resources were offered on preparation for the first period, and on fertility and pregnancy.

April 22

Von Willebrand Disease (vWD) was focused on, reviewing questions such as dental care for people with vWD, physiotherapy and joint bleeds, as well as the treatment with the DDAVP.

Open the Door to Another Reality

EHC MULTIMEDIA EXHIBITION

In 2020, to celebrate the 30th anniversary of the EHC, a multimedia exhibition “Open the Door to Another Reality” was created, literally offering people the possibility to open a door and listen to the stories of people affected by bleeding disorders over different times and places across eight decades, and hear their hopes and visions for the future.

Originally foreseen to launch on 24 April 2020 to coincide with the inauguration of the EHC’s new office, due to the COVID-19 shutdown, the exhibition was launched alongside the EHC Leadership Conference in Brussels in June 2022.

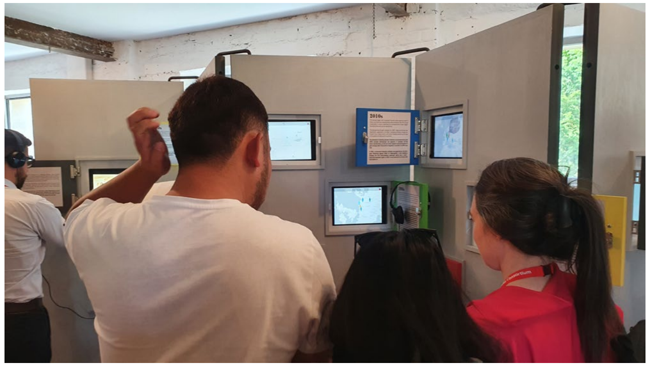
When building the exhibition, the idea of building a shared (hi)story was in mind and therefore the EHC warmly invites its NMOs to welcome the exhibition at their events, so that members of the bleeding disorders community across the EHC membership can witness the history of the bleeding disorders community and add their own stories!

OPEN THE DOOR TO ANOTHER REALITY

INTRODUCTION

View video on **https://youtu.be/WCUTfvFIVxs**

View the exhibition webpage:
www.ehc.eu/library/multimedia/ehc-exhibition



EHC Conference 2022

ADVOCACY AND AWARENESS

To advocate and partner effectively towards improving quality of life for all people with congenital bleeding disorders in Europe.

EHC Conference 2022

From 6-9 October 2022, the EHC held its Conference and General Assembly in Copenhagen, Denmark. This annual event was an opportunity for the bleeding disorders community, both patients and healthcare providers, to hear the latest updates on the treatment and care of various bleeding disorders, as well as avail of the opportunity of informal exchange and community building.

This event was organised in a hybrid format, allowing attendees to join from home, if they were not able to travel.

The programme of the conference focused on the following topics:

- Danish experience of bleeding disorder treatment;
- Mild and moderate bleeding disorders;
- Shared decision-making and patient education;
- Women with Bleeding Disorders;
- Von Willebrand Disease;
- Non-replacement therapies, and reporting and monitoring for existing and new therapies;
- Liver health

The SLAMS session offered short glimpses on unique projects carried out in the areas in treatment, care and on the level of the patient community. Among others, the projects presented included the concept of a carousel clinic, physiotherapy outside the haemophilia treatment centres and identification of bleeding disorder patients in emergency situations.

The traditional EHC Youth Debate took place and the debaters challenged each other on the topics of:

- Debate 1: With advancing therapies, reductions in bleeds, better joints and outcomes - should we focus on developing centres of excellence or invest in telemedicine?
- Debate 2: Centres of Excellence are home to expertise, but local centres are home to a life-long relationship with healthcare providers. What is more important - trust or expertise?

Besides the Conference, several side events took place before and during this main event.

Two pre-conference workshops were held on 5 October - one in the framework of EHC von Willebrand Disease Platform and other as a part of European Rare and Inhibitor Network (ERIN) programme. The participants of both workshops also got the opportunity to interact and together discuss their needs and shared challenges.

The EHC Volunteers Mixer was introduced for the first time and was hugely successful. This satellite event brought together the volunteers of the many EHC committees and working groups, and served as an opportunity to exchange about the objectives and work of each of the groups, as well as best and worst practices, this way strengthening the sense of working towards joint goals and being part of the same inclusive bleeding disorders community.

In addition, the nurses workshop on Sexual Health and Intimacy was held on 7 October, focusing more in detail on the role of nurses in approaching the questions of sexual health and intimacy, sharing the best practices and experience, as well as discussing the "appropriate" language and right questions to ask.

EHC THINK TANK

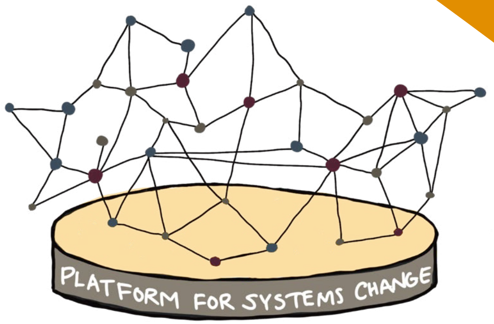
In 2022, the EHC Think Tank hosted 12 workshops for the three running workstreams on registries, the hub & spoke model, and patient agency. Over the course of the year, all workstream participants collaborated, listened, and actively engaged in trying to uncover the structures, patterns, and mental models underlying their respective thematic areas and potentially feeding into current challenges.

You can find more information about each of the workstreams below and on the EHC Think Tank website.

In tandem with the workstreams, we ran an innovation lab consisting of surveys, workshops, and meetings undertaken to learn from different stakeholders about their perspectives on relevant themes to tackle in the EHC Think Tank workstreams. This led to two new workstreams on access equity and future care pathways, respectively, which were launched at the inspirational virtual public event in June, which gathered more than 50 people.

At the organisational level, we grew our internal staff base and launched our own website with information about the thematic work and articles about key concepts, e.g., social innovation, behavioural science, and systems change.

The Think Tank is constantly evolving and received outstanding guidance from its Council and its Faculty in terms of thematic focus and utilising methodologies.



WORKSTREAMS

REGISTRIES

This workstream seeks to untangle the complexities of registries, to find common ground, and to co-construct a collectively endorsed forward direction, particularly in the areas of:

- The role of the patient in data collection
- The role of healthcare providers in data collection
- Data quality
- Data sharing
- Multiplicity of registries
- Lack of common purpose
- Sustainable funding to develop and maintain registries

THE HUB & SPOKE MODEL

The hub & spoke model workstream explores future treatment pathways, building on international calls for a hub-and-spoke model approach, particularly in terms of how to manage:

- Delivery of gene therapy
- Resource allocation
- Criteria for hubs and spokes
- Collaboration between hub and spokes
- Knowledge distribution
- Re-envisioning delivery of treatment in the future

PATIENT AGENCY

The workstream on patient agency addresses how to meaningfully embed and integrate patient expertise up-front and throughout the design, organisation, implementation, and evaluation of treatment and care, as well as capturing and utilising patient preferences.

Besides working on a definition of patient agency incorporating behaviour, the group identifies the patient's role as a key challenge to ensure that healthcare is collaborative and based on the patient's ability to make choices.

ADVOCACY AND AWARENESS

To advocate and partner effectively towards improving quality of life for all people with congenital bleeding disorders in Europe.

OUTCOMES:

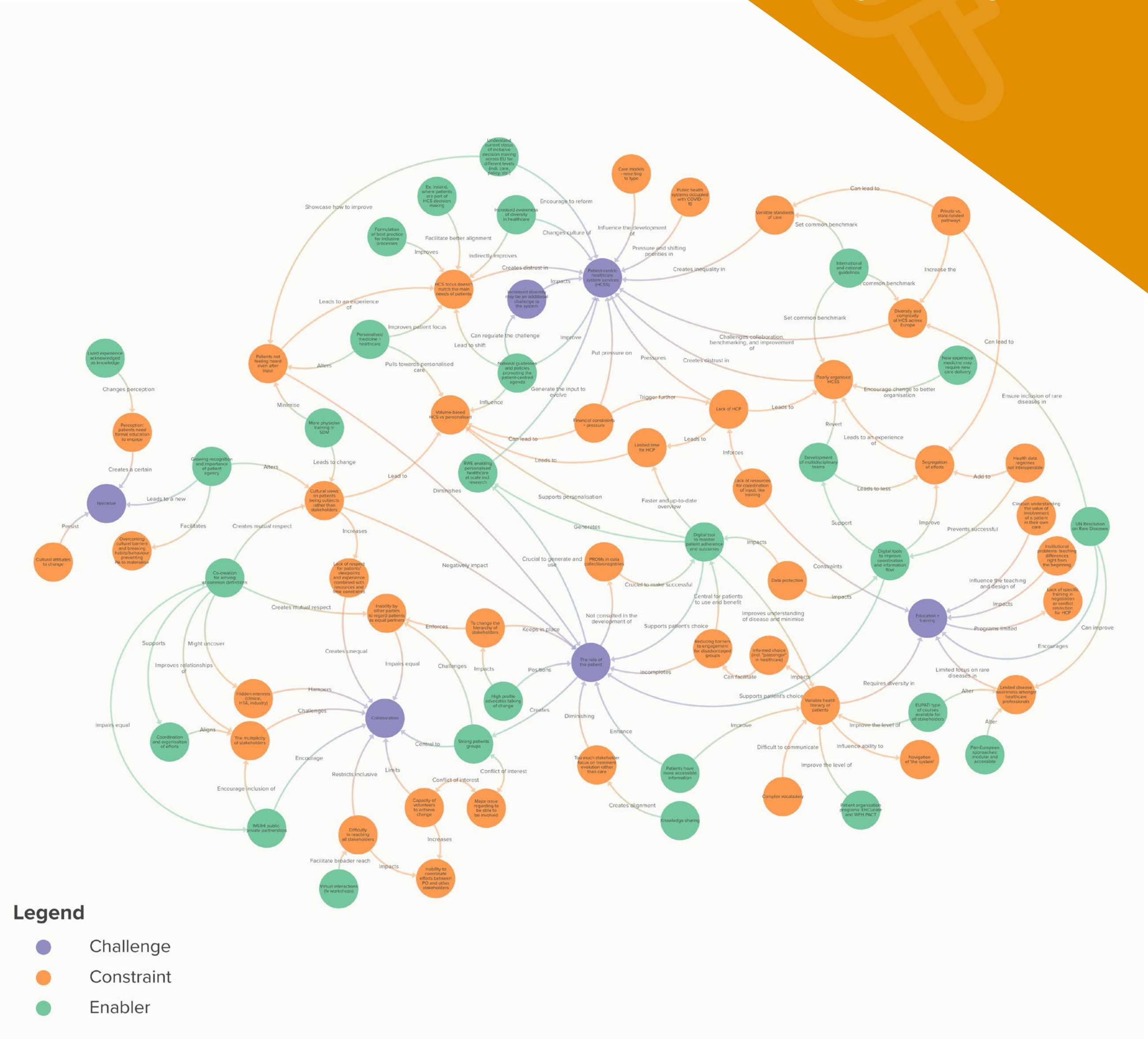
- “Registry Mapping – An overview of rare bleeding disorder registries across Europe” by Copenhagen Economics
- “Targeted literature review – Understanding the description and application of patient agency in the healthcare context” by OPEN Health and
- “Gene Therapy Landscape” by OPEN Health

PUBLICATIONS

“Key challenges for patient registries – A report from the 1st workshop of the EHC Think Tank Workstream on Registries” *The Journal of Haemophilia Practice*, vol.9, no.1, 2022, pp.14-19.

Bok, Amanda, Noone, Declan and Skouw-Rasmussen, Naja. “Patient agency: key questions and challenges – A report from the 1st workshop of the EHC Think Tank Workstream on Patient Agency” *The Journal of Haemophilia Practice*, vol.9, no.1, 2022, pp.27-35.

Bok, Amanda, Noone, Declan and Skouw-Rasmussen, Naja. “Key challenges for hub and spoke models of care – A report from the 1st workshop of the EHC Think Tank on Hub and Spoke Treatment Models” *The Journal of Haemophilia Practice*, vol.9, no.1, 2022, pp.20-26.



ADVOCACY AND AWARENESS

To advocate and partner effectively towards improving quality of life for all people with congenital bleeding disorders in Europe.

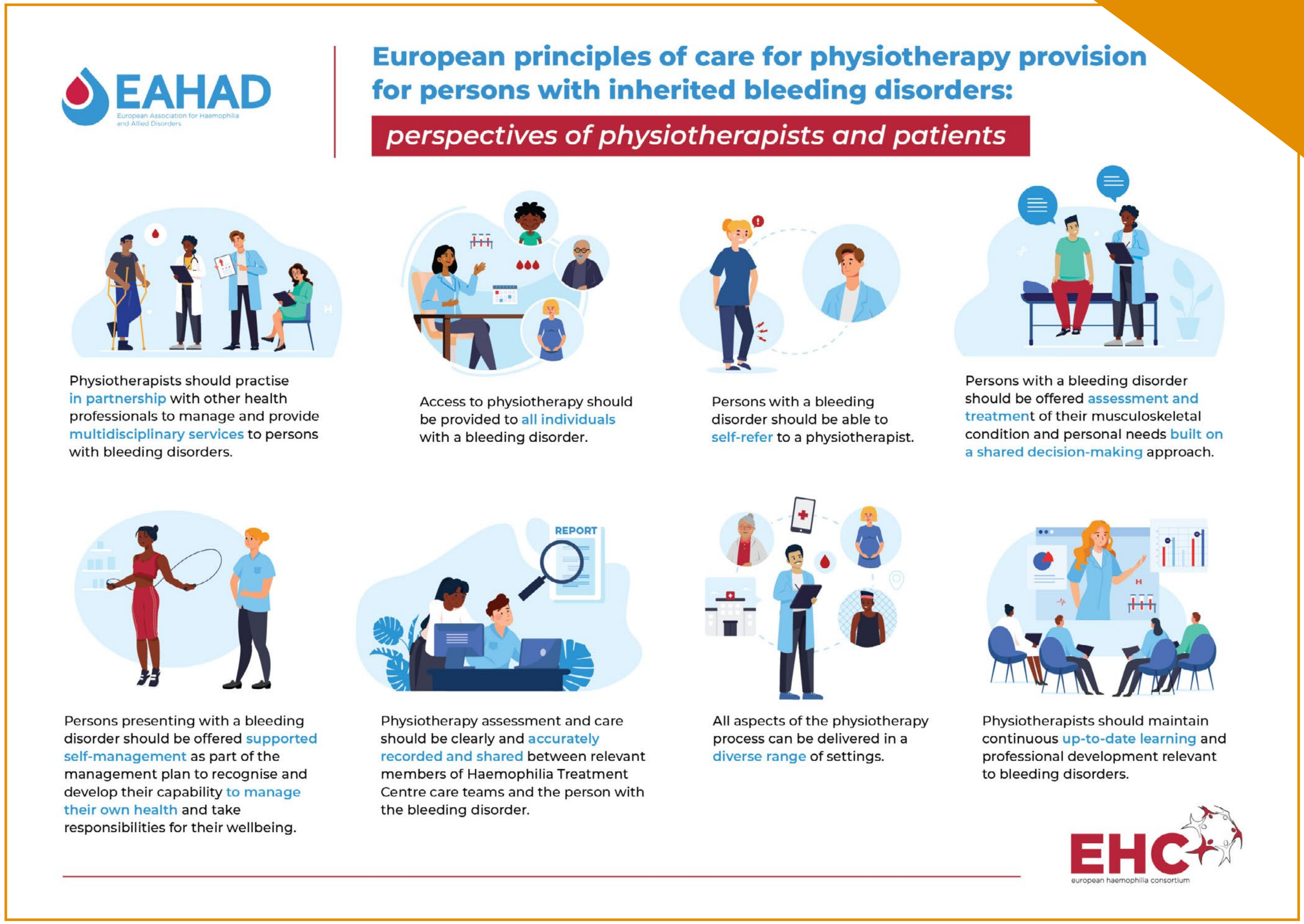
European principles of care for physiotherapy provision for persons with inherited bleeding disorders:

Perspectives of physiotherapists and patients

In May 2022, an article **"European principles of care for physiotherapy provision for persons with inherited bleeding disorders: Perspectives of physiotherapists and patients"** was published in the scientific journal *Haemophilia*. This publication was a result of collaboration between the Physiotherapists Committee of the European Association for Haemophilia and Allied Disorders (EAHAD) and patient representatives from the side of the EHC. The paper argues that access to high quality individualised physiotherapy should be ensured for all people with bleeding disorders, including those with mild and moderate severities, male and female, people with von Willebrand Disease (vWD) and other rare bleeding disorders. Physiotherapy should be viewed as a basic requisite in their multidisciplinary care.

To facilitate the use of this publication for patient advocacy, an infographic summarising the principles was subsequently created and distributed within the EHC and EAHAD networks. The infographics are available for the translation upon request.

The full article can be accessed here:
onlinelibrary.wiley.com/doi/10.1111/hae.14566



EAHAD
European Association for Haemophilia and Allied Disorders

European principles of care for physiotherapy provision for persons with inherited bleeding disorders:

perspectives of physiotherapists and patients

Physiotherapists should practise in partnership with other health professionals to manage and provide multidisciplinary services to persons with bleeding disorders.

Access to physiotherapy should be provided to all individuals with a bleeding disorder.

Persons with a bleeding disorder should be able to self-refer to a physiotherapist.

Persons with a bleeding disorder should be offered assessment and treatment of their musculoskeletal condition and personal needs built on a shared decision-making approach.

Persons presenting with a bleeding disorder should be offered supported self-management as part of the management plan to recognise and develop their capability to manage their own health and take responsibilities for their wellbeing.

Physiotherapy assessment and care should be clearly and accurately recorded and shared between relevant members of Haemophilia Treatment Centre care teams and the person with the bleeding disorder.

All aspects of the physiotherapy process can be delivered in a diverse range of settings.

Physiotherapists should maintain continuous up-to-date learning and professional development relevant to bleeding disorders.

EHC
european haemophilia consortium

Partnerships

The advocacy and awareness raising work of the EHC would not be possible without the various partnerships and collaborations pursued.

World Federation of Hemophilia (WFH)

www.wfh.org

European Association for Haemophilia and Allied Disorders (EAHAD)

www.eahad.org

EURORDIS-Rare Diseases Europe

www.eurordis.org

European Patients' Forum (EPF)

www.eu-patient.eu

European Association for the Study of the Liver (EASL)

easl.eu/patient-synergies

International Society on Trombosis and Haemostasis (ISTH)

www.isth.org

Platform of Plasma Protein Users (PLUS)

plasmausers.org

European Hematology Association (EHA)

ehaweb.org

Transform Alliance

transformalliance.eu

In 2022, the EHC attended the following key events:

23-24 January
GHAL (Global Haemophilia Advocacy Leadership) Summit

2-4 February
EAHAD Congress, virtual

4-6 March
IHS (Irish Haemophilia Society) Congress

11-13 March
Nordic-Baltic Meeting, Oslo, Norway

15 March
Together for Rare Diseases multi-stakeholder meeting

22-24 March
EPA Conference

31 March
TRANSFORM MEP Interest Group

1 April
Multistakeholder workshop on EMA's extended mandate

23 April
EPF General Assembly

9-11 May
WFH World Congress Montreal

18 May
EURORDIS GA

25 May
EUnetHTA 21 Roundtable with Patients and Healthcare Professionals

14 June
Together4RD - 2nd Multi-stakeholder meeting

16 June
TRANSFORM MEP Interest Group: The Regulatory Framework for ATMPs

23-24 June
EPF Congress

27 June - 1 July
ECRD: 11th European conference on rare disease & orphan products

July 9-13
ISTH Congress in London

1 September
EESC Ensuring strong European solidarity for rare disease patients

28-29 September
European Health Forum Gastein

28 September
EP STOA workshop 'Creation of a public European medicines infrastructure: Purpose and feasibility'

17 October
Virtual World Health Summit Roundtable: Realizing the Promise of Gene Therapy

26 October
FEAM Forum Annual Lecture Digital Health and AI

27-28 October
WFH Global Policy & Access Summit

28 October
EPF AI Webinar - EU Legislation on AI

14 November
EURORDIS Roadmap for Guideline Implementation Webinar

17-18 November
5th WFH Gene Therapy Round Table

30 November
Belgian Impact Day

8 December
EFPIA European Health Summit 2022

As of December 2022, the EHC is also represented in two of the European Patients' Forum (EPF) working groups:

- Working Group on Universal Access to Healthcare that informs, guides and supports the work of EPF in advocating for universal access to healthcare for all patients.
- Working Group on Digital health that contributes to developing EPF's overall strategy in digital health, including the protection and sharing of data from patients' electronic health records.

EDUCATION ENGAGEMENT DEVELOPMENT

To ensure state-of-the-art knowledge and relevant skills for National Member Organisation (NMO) representatives to help them achieve better treatment and comprehensive care nationally.

EHC Educational App EHCucate

An accessible patient-centred educational resource which evolves apace of the treatment landscape.

In the past few years, the rare bleeding disorders community has been facing the emergence of novel and highly complex therapies. In 2020, the EHC decided to launch an educational project which together with its traditional advocacy activities would be a key element for individual treatment journeys of our patients. As a result, the EHC created an educational app incorporating all levels of educational information about therapies, ranging from basic to advanced, and uses a variety of in-app media to be engaging to a highly heterogeneous audience.

EHCucate is available for all and for free, both on Google Play (Android) and App Store (iOS). While the treatment landscape of inherited BDs is evolving rapidly with complex therapeutic options, the EHC therapies educational app offers people with inherited bleeding disorders (PwIBD) the opportunity to personalise their exploration of this landscape, empowering them with the knowledge and confidence to continue to partner with healthcare providers in its navigation.

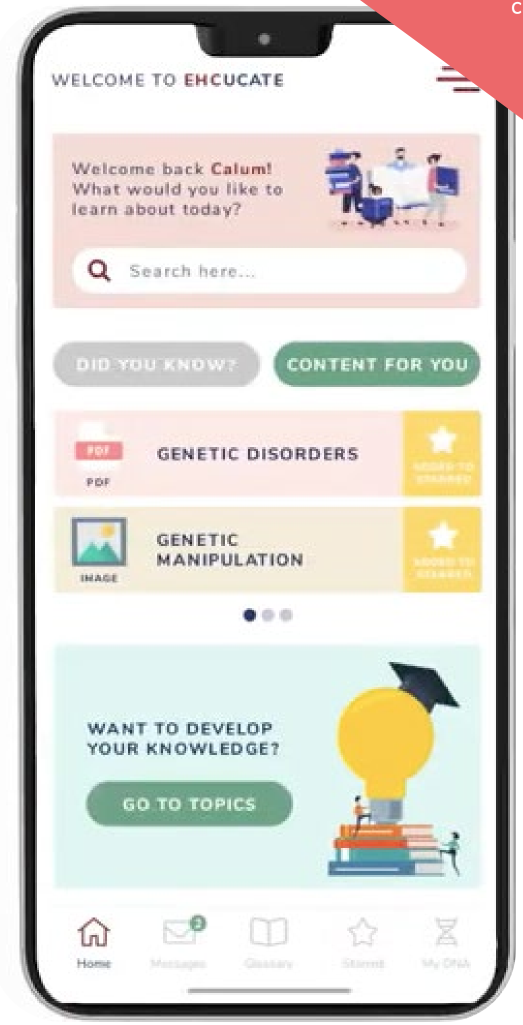
The app features a progressive topic framework, building a detailed understanding of all therapy options, including complex novel therapies, upon a solid basic knowledge foundation. Multiple learner-driven content exploration pathways, interactive features, and creative presentations (e.g., an original rap video) enhance learner engagement. Self-assessment activities, custom medical illustrations/animations, deep-dive resources, and personalised reference collections empower learners to individualise their interaction with the app to meet their needs.

Designed to facilitate frequent content updates and additions, modifications are actively integrated into existing app content, making them instantaneously available without any action required by the learner.

Scan the QR codes to download the app and be on top of the information:

for Android

For iOS


WELCOME TO EHCUCATE

Welcome back Calum! What would you like to learn about today?

New Technologies Workshop | 11-13 November 2022

To ensure state-of-the-art knowledge and relevant skills for National Member Organisation (NMO) representatives to help them achieve better treatment and comprehensive care nationally.

New Technologies Workshop

11-13 NOVEMBER 2022

In 2022, the EHC Workshop on New Technologies in Haemophilia and Other Rare Bleeding Disorders went back to a fully face-to-face format and close to 80 participants gathered in Marseille, France.

This event is key to providing EHC members with education and information on the latest developments in novel therapies.

In addition to the sessions on replacement and non-replacement therapies, as well as gene therapy, the treatment and care of people living with extremely rare bleeding disorders was discussed. In terms of organisation of care, sessions on shared decision-making and financial considerations regarding different treatment options were held.

The programme of the EHC New Technologies Workshop was developed by the EHC Steering Committee and Medical Advisory Group with the support of EHC Public Policy and Communications Officer Laura Savini.

EDUCATION ENGAGEMENT DEVELOPMENT

To ensure state-of-the-art knowledge and relevant skills for National Member Organisation (NMO) representatives to help them achieve better treatment and comprehensive care nationally.

EHC Workshop on Health Economics

9-11 SEPTEMBER 2022

A big part of the work of the EHC's National Member Organisations (NMOs) consists of ensuring adequate access to treatment and care for people living with all bleeding disorders in their country.

In order to successfully carry out this work, the NMOs need to be empowered and equipped with the necessary tools to improve access to treatment in their communities.

To this end, the EHC regularly organises a workshop that looks at various aspects of health economics, such as using the data and information on different treatment products available, and working with payers and regulators. The workshop is tailored to a concrete group of the NMOs each time, so that the content and discussions can be more useful for their specific contexts. In 2022, the workshop thematically focused on Tenders and Procurement and took place in Malmö, Sweden.

The programme was composed of lectures, panel discussions and practical group exercises, and thematically looked at the updates regarding treatment for various bleeding disorders, mechanisms for pricing and reimbursement, as well as looking at various economic models and finding the best fit for each country.

The programme of the EHC Workshop on Health Economic was developed by the members of EHC Steering Committee and EHC Medical and Scientific Advisory Group with the support of the EHC Public Policy and Communications Officer Laura Savini.

EDUCATION ENGAGEMENT DEVELOPMENT

To ensure state-of-the-art knowledge and relevant skills for National Member Organisation (NMO) representatives to help them achieve better treatment and comprehensive care nationally.

Novel Treatment Products Newsletter

With so much change in the therapeutic landscape in rare bleeding disorders, it is critical to ensure that EHC members are kept abreast of all relevant scientific and clinical developments.

The Novel Treatment Products Newsletter covers recent major developments and is divided into different types of disorders for which there is an update to report. The information provided in this newsletter is compiled from multiple sources, including presentations at recent scientific meetings, websites, financial information and by writing directly to pharmaceutical companies. It is then redrafted and presented in easy-to-understand language. The EHC issues this publication twice a year in English.

The EHC welcomes all treatment developments that may benefit patients in the future. The EHC takes no position on any product type or class reported in this newsletter.

Here you can read all issues of EHC Novel Treatment Products Newsletter:

Consult all the issues of the newsletter here:
www.ehc.eu/novel-treatment-products-newsletter

#thisway campaign

In the first half of 2022, the #thisway campaign was on a hiatus from physical activities, but it was resumed in the second half with a guided tour at the EHC conference in Copenhagen which included over 30 participants, and a podcast looking at the aspects of wellbeing that go beyond the physical activity was published on the EHC Soundcloud page. It featured a conversation with wellbeing expert Ilze Jēče, giving a lot of food for thought and something in it for everybody - patients, parents, women, youth, older generation, NMO leaders, NMO volunteers.

Listen to the podcast on Souncloud:
soundcloud.com/european-haemophilia-consortium/thisway-beyond-the-physical-aspect-of-wellbeing

New Products Working Group

The EHC is grateful for the work and support of the New Products Working Group, which has overseen the content and production of this newsletter. In 2022, the following volunteers were members of this committee:

- **Mariëtte Driessens** | Dutch NMO
- **Radoslaw Kaczmarek** | Polish NMO
- **Ilmar Kruis** | Dutch NMO
- **Declan Noone** | EHC President, Irish NMO
- **Brian O'Mahony** | EHC MASAG, Irish NMO
- **David Page** | Canadian Hemophilia Society
- **Geniève Piétu** | French NMO
- **Flora Peyvandi** | EHC MAG, Italy
- **Uwe Schlenkrich** | German NMO
- **Michael Makris** | Chair, EHC MAG, United Kingdom
- **Dan Hart** | EHC MASAG, United Kingdom

In addition to these volunteers, the EHC Novel Treatment Products Newsletter is also edited by some of the EHC MAG and MASAG members. The work of this committee was supported by Laura Savini, EHC Public Policy and Communications Officer.

COMMUNITY EMPOWERMENT

To empower NMOs and support their future development

EHC Youth Leadership Workshop 2022

EHC Youth Leadership Workshop

8-10 APRIL 2022

After 2 years of delivering the Youth Leadership Workshop virtually, the EHC were delighted to have our YLW face-to-face in Amsterdam on April 8th - 10th 2022.

The workshop brought together 15 participants from 10 countries - Slovenia, Ireland, Serbia, Malta, Croatia, Bulgaria, Greece, Bosnia & Herzegovina, the UK and Azerbaijan. As always, the YLW programme focused on a mix of lecture and interactive exercises covering volunteering, internally and externally communication and collaboration, as well as providing an overview of medicines development and the role of industry in the community.

Youth Fellowship Programme

Despite the challenges of the COVID-19 pandemic and associated confinement, the EHC felt strongly that we needed to continue to engage with and invest in our youth and the future generations of leadership for our community and NMOs. Our Youth Fellowship Programme is intended to bring youth representatives in our community to work together cross-nationally on common projects. We encouraged our youth to engage with each other and stay connected online when face-to-face encounters were not possible. We held several virtual calls throughout the year with the purpose of touching base, connecting, and planning for future programmes.

Youth Committee

The EHC Youth Committee provides strategic guidance to the EHC on its Youth Programme. The Youth Committee also develops and delivers the agendas and content of the EHC's youth training programmes and supervises EHC Youth Fellowship Programmes.

In 2022, the EHC Youth Committee was composed of:

- **Aizat Aydarbekova** | Kyrgyz NMO
- **Marija Aloa Vera Drzmanoska** | North Macedonian NMO
- **William McKeown** | UK NMO
- **Olivia Romero-Lux** | EHC Youth Expert, French NMO
- **Stefan Radovanovic** | EHC SC, Serbian NMO

The work of the EHC Youth Working Group is supported by Fiona Brennan, EHC Community Programmes Officer.

EHC Leadership Conference

16-19 JUNE 2022

The EHC Leadership Conference is an essential element of strengthening the bleeding disorders community. This event brings together key figures of the EHC National Member Organisations (NMOs) - senior NMO leaders, young NMO leaders and NMO staff - in an informal setting to discuss issues that are important to them, as well as share experiences and best practices, to learn and grow together.

In 2022, the Leadership Conference was held as a face-to-face meeting in Brussels after two years as an online event.

The programme of the event consisted of various interactive and participatory methods and focused on the themes, such as new developments in bleeding disorder treatment, including gene therapy, as well as the future of bleeding disorder care.

Given that the Leadership Conference is an important platform for the NMOs to share their experiences and best practices, sufficient time was allocated in the programme for know-how and workshop sessions. Those sessions focused on tools for patient and community engagement, in particular on shared decision-making, Principles of Care as a tool for advocacy, as well as the EHC Think Tank and Community Advisory Boards as different approaches to patient advocacy and engagement with pharmaceutical industry. In addition, aspects of working with youth in a post-pandemic setting, language as the first step towards inclusion, as well as tools and ideas for working with and for women were explored.

In order to explore the theme of patient education, three concrete projects were presented: Expert Patient initiative from the French NMO; Role of patients in education of doctors - an educational approach from Romania, carried out by EHC SC member Diana Lighezan; and the EHCucate educational app of the EHC as an accessible and interactive way of delivering patient education.

Special attention was given to the efforts and the responses of solidarity from the community during the past year - one that has been difficult for the society in general, with the post-pandemic setting and in particular the war in Ukraine, and that put the bleeding disorders community under tremendous pressure.

A session focusing on wellbeing was offered to the participants to share their challenges and help explore ways to support the volunteers and staff of the NMOs through straining times.

Finally, the young generation of the bleeding disorders community was put in the limelight, together with esteemed leaders of the community, discussing ways of growing into a role of a leader, but also the ways of growing out of different roles along the way.

The programme of the EHC Leadership Conference was developed by the EHC Steering Committee with the support of EHC Community Programmes Officer Kristine Jansone and based on the conversations with the NMOs and accessing their objectives and needs.

European Women and Bleeding Disorders Conference

20-22 MAY 2022

In 2022, after two years of strong online engagement with the community, the second EHC Conference on Women with Bleeding Disorders took place in a hybrid format, with more than a hundred attendees joining face-to-face in Basel, Switzerland, and a few dozen more online.

In 2022, after two years of strong online engagement with the community, the second EHC Conference on Women with Bleeding Disorders took place in a hybrid format, with more than a hundred attendees joining face-to-face in Basel, Switzerland, and a few dozen more online.

During the first day of the conference, the programme focused on Quality of Life, featuring sessions on Principles of Care for Women and Bleeding Disorders and Practical tips for patients and clinicians for the management of bleeding disorders in women and girls, as well as discussing ways to monitor improvements of their quality of life. The day was concluded with the "Cocktail talks" that allowed participants to exchange on topics important to them in an informal setting.

The second day of the conference was dedicated to the patient life cycle, looking at issues that women with bleeding disorders face in different stages of their lives, featuring sessions dedicated to psychosocial issues and ageing with a bleeding disorder. Furthermore, time was allocated for peer-to-peer sessions of participants of various age groups. At the end of the second day, the film "Mulheres" produced by the National Member Organisation in Portugal was screened.

Watch the film "Mulheres" here:
https://youtu.be/rLQOqA7xBT4?feature=shared

The final day of the conference discussed comprehensive care for women and girls with bleeding disorders, looking specifically at musculoskeletal health, fertility and iron deficiency. Finally, a session on advocacy was held to support and equip the participants in their efforts to advocate for women and girls with bleeding disorders.

COMMUNITY EMPOWERMENT

To empower NMOs and support
their future development

The proceedings of the 2022 EHC Conference on Women and Bleeding Disorders are available as open-access articles in the Journal of Haemophilia Practice. Click on the links below to read:

- **Conference introduction**,
- **Monitoring to improve quality of life for women with bleeding disorders**,
- **Ageing and women with bleeding disorders,**
- **Focus on musculoskeletal health in women and bleeding disorders,**
- **The Cinderella Study, women's lived-in experience with bleeding disorders – a CSL Behring Symposium,**
- **Advocacy worldwide for women with bleeding disorders.**

In advance of the Conference on Women with Bleeding Disorders, a practical pre-conference workshop for women with bleeding disorders took place on 19 May. It focused on problem-solving, advocacy, conflict management and negotiation, as well as developing a media plan. In addition, case studies and best practices from various NMOs were presented.

WBD Committee

The objective of the EHC Women and Bleeding Disorders Committee is to improve the quality of life of women and girls impacted by bleeding disorders. The committee creates awareness, recognition, support, and education about, and for, women in the bleeding disorder community at the European level. In 2022, the EHC Women and Bleeding Disorders Committee was composed of:

- **Evelyn Grimberg (chair)** | Dutch NMO
- **Marion Bräuer** | Austrian NMO
- **Yannick Collé** | French NMO
- **Karola Diósi** | Hungarian NMO
- **Diana Lighezan** | EHC SC, Romanian NMO
- **Tatjana Markovic** | Serbian NMO
- **Katja Peltoniemi** | Finnish NMO
- **Anna Tollwé** | Swedish NMO

The work of the EHC WBD Committee is supported by Laura Savini, EHC Public Policy and Communications Officer.

WBD Ambassadors

As part of the effort to increase awareness and support members in the NMOs, the WBD Committee has established a network and hosted an online session to exchange information and discuss local advocacy efforts.

WBD Committee Retreat

11-13 March 2022

The Women and Bleeding Disorders Committee gathered in Basel, Switzerland over the weekend to advance their work on improving access to diagnosis and treatment for women with bleeding disorders, and to finalise the last details for the second European Women and Bleeding Disorders Conference.

Von Willebrand Disease

The VWD Working Group met in January 2022 to put together an action plan for their work and contribution to the European VWD Platform for 2022 & 2023. The retreat also including training for the working group members on advocacy and interactions with internal & external stakeholders. Throughout 2022 the following resources and activities were developed as part of the ongoing work to support, education and create awareness for people with VWD across Europe:

- Infographics on the International Guidelines developed for both patients and NMOs
- Resources including VWD booklet & podcasts with clinicians and patients shared with NMOs
- Pre-conference workshops for patients and NMO leaders organised in May 2022 & October 2022
- Information sessions with experts organised for VWD WG to empower and educate
- Undergoing development of Principles of Care with clinician experts and patient perspectives
- VWD Survey submitted for publication
- International dental workshop on VWD organised and supported by EHC in August 2022

Pre-Conference VWD Workshop

The VWD Working Group organised a pre-conference workshop for patients with VWD and NMO leaders in conjunction with the Women with Bleeding Disorder Conference in May 2022 in Basel, Switzerland. The workshop was attending by 9 participants who worked together on issues faced by the VWD community. The group also received a medical update and training on negotiation and conflict management.

VWD Working Group

The VWD Working Group was set up in 2020 to lead the EHC VWD Platform for Europe. This platform promotes the formation of a European community of People with von Willebrand Disease (PwVWD) enabling them to find support and 'identity' while also promoting, building and maintaining a network of European VWD advocates. The VWD Working Group maintained their regular monthly online meetings while also planning and delivering the face-to-face workshops for 2022. The group reviewed and provided extensive feedback on key advocacy tools and resources for the VWD community. In December 2022, the von Willebrand Disease (VWD) Working Group convened to develop an action plan for their involvement in the European VWD Platform for 2023. During this meeting, the WG also decided to seek a status change and requested the SC's approval to transition into a Committee, reflecting the significant progress achieved in previous years.

In 2022, the EHC Von Willebrand Disease Working Group was composed of:

- **Shannon Carey** | Irish NMO
- **Manon Degenaar-Dujardin** | Dutch NMO
- **Marianna Kladi** | Greek NMO
- **Donal McCann** | Irish NMO
- **Julia Rauscher** | Austrian NMO
- **Joanne Traunter** | UK NMO
- **Baiba Ziemele** | Latvian NMO
- **Sunny Mainir** | UK NMO

The work of the VWD WG is supported by Fiona Brennan, EHC Community Programmes Officer.

European Rare and Inhibitor Network (ERIN)

In 2021, the achievements of the European Inhibitor Network (EIN) were evaluated and a conclusion was made that largely the initial objectives have been met and it is time to set new goals and to capitalise on the achievements of the network.

It was decided to capitalise on the experience and success of the inhibitor community and create a space to build the community of people living with extremely rare bleeding disorders, thus the European Rare and Inhibitor Network (ERIN) was born and started its first activities in 2022.

In the beginning of 2022, the transition into the European Rare and Inhibitor Network (ERIN) began - impact assessment and revision of activities in the inhibitor part plus initial plan for very rare, process for focus group started and surveys were developed.

The objectives of the ERIN are:

1. To better understand and prioritise the needs of people with extremely rare bleeding disorders both at a community level as well as at a policymaking level;
2. To empower people with extremely rare bleeding disorders to drive the research in the field of extremely rare bleeding disorders thus taking steps to improve the treatment and care for these patient cohorts;
3. To enable social and intellectual connections between people with extremely rare bleeding disorders and between their respective caregivers to allow them to mutually support and educate one another;
4. To ensure that people with extremely rare bleeding disorders themselves are also active and effective at engaging directly with decision-makers to ensure that they receive the treatment and care that they need; and
5. To continue offering the necessary support to people with inhibitors and their caregivers, ensuring the transfer of knowledge to local inhibitor communities.

Inhibitor Working Group

The planning and activities of ERIN were coordinated by the Inhibitor Working Group (IWG) - a group of volunteers, consisting of patients, parents, healthcare professionals, and patient organisation representatives. The IWG is in charge of overseeing the ERIN programme, and it collaborates closely with the EHC NMOs. In 2022, the IWG regularly met online to plan, implement, and evaluate the activities carried out for the inhibitor and extremely rare bleeding disorders community. In 2022, the EHC Inhibitor Working Group was composed of:

- **Enea Atroce** | Swiss NMO
- **Christina Burgess** | United Kingdom
- **Miguel Crato** | EHC SC, Portuguese NMO
- **Mirko Jokic** | Serbian NMO
- **Ioana Malita** | Romania
- **Dr Maria Elisa Mancuso** | Italy
- **Jim O'Leary** | Irish NMO
- **Teresa Pereira** | Portuguese NMO

At the end of 2022, the term of the Inhibitor Working Group ended, giving the space for the ERIN Committee that would continue the work in 2023. The EHC is immensely grateful to all the members of the IWG for their contribution to the success of the European Inhibitor Network and European Rare and Inhibitor Network. The work of the EHC IWG was supported by Kristine Jansone, EHC Community Programmes Officer.

COMMUNITY EMPOWERMENT

To empower NMOs and support their future development

BUILDING THE EXPERTISE - FROM KNOWLEDGE TO ACTION

ERIN on-line training session

WHEN? 5th and 6th September 2022 from 16:00-18:00 CET

WHAT? Day 1: Treatment, multidisciplinary care and shared decision-making // Day 2: Patient engagement, activism and advocacy training.

HOW? This will be an interactive on-line event

WITH Dr Maria Elisa Mancuso, Miguel Crato & Kristine Jansone

ERIN

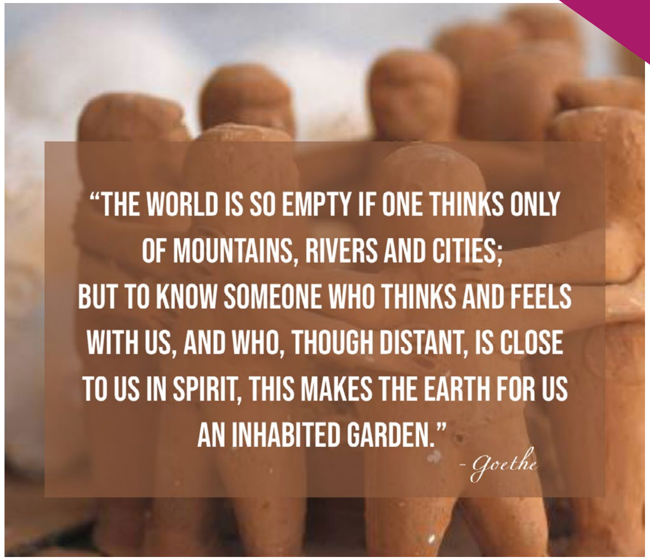
Online Training Sessions for Inhibitor Community

To ensure the transfer of knowledge from the inhibitor community on the European level to the local communities, two sets of online training sessions titled “Building an Expertise - From Knowledge to Action” were held in 2022:

- On 30 and 31 May - sessions in Russian;
- On 5 and 6 September - sessions in English.

The programme of these training sessions was divided into two blocks: Treatment, multidisciplinary care and shared decision-making, and Patient engagement, activism and advocacy training. The first block featured themes, such as looking for veritable information on treatment and care, and shared decision-making, whereas the second block focused on patient engagement and advocacy training.

In advance of the Conference on Women with Bleeding Disorders, a practical pre-conference workshop for women with bleeding disorders took place on 19 May. It focused on problem-solving, advocacy, conflict management and negotiation, as well as developing a media plan. In addition, case studies and best practices from various NMOs were presented.



“THE WORLD IS SO EMPTY IF ONE THINKS ONLY OF MOUNTAINS, RIVERS AND CITIES; BUT TO KNOW SOMEONE WHO THINKS AND FEELS WITH US, AND WHO, THOUGH DISTANT, IS CLOSE TO US IN SPIRIT, THIS MAKES THE EARTH FOR US AN INHABITED GARDEN.”

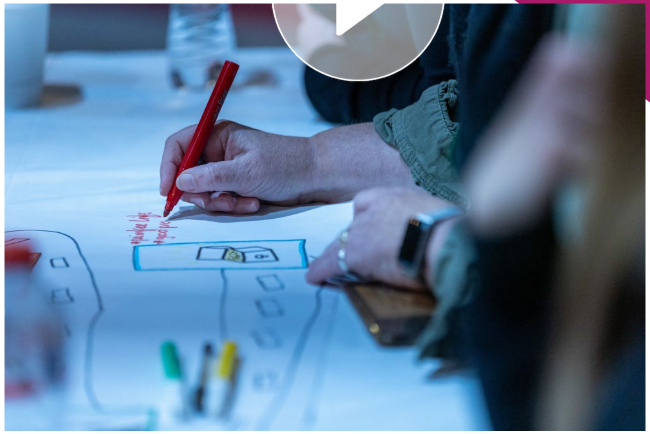
- Goethe

Peer-to-Peer Sessions

To continue offering support to the inhibitor community, the online peer-to-peer sessions were held throughout 2022. On a quarterly basis, an hour-long session for adults with an inhibitor, parents of children with inhibitors and partners of adults with inhibitors. To facilitate participation of the participants from the Russian-speaking National Member Organisations, interpretation into Russian was provided during the sessions.

Needs Assessment Survey

This extended programme was started off by a needs assessment survey among people living with extremely rare bleeding disorders across Europe. The survey revealed that among the challenges that people living with extremely rare bleeding disorders are lack of information regarding their bleeding disorder, isolation, psychological and emotional discomfort, as well as limited access to treatment (including treatment options) and diagnosis. Also, having no proper information from haematologists following diagnosing or no information about the research into novel treatment options were mentioned. This survey offered good material to build the ERIN programme.



COMMUNITY EMPOWERMENT

To empower NMOs and support
their future development

ERIN Summit

ERIN Summit

1-4 DECEMBER 2022

In 2022, the ERIN Summit, bringing together patients living with inhibitors and extremely rare bleeding disorders, and their families for the first time was held in Barretstown, Ireland.

The programme contained sessions for each of the patient cohorts, offering latest updates on the treatment and care, as well as special time and space for answering their questions and discussing. Given that the multidisciplinary care is highly important to all people living with a bleeding disorder, several sessions were dedicated to this topic, through exploring the importance of movement and different approaches needed for each bleeding disorder, as well as looking at nursing care, dental care and psychosocial care in the patient cohort-specific setting.

Traditionally, an important part of the summit is peer-to-peer sessions, therefore enough time was given for peer exchange.

Together, all participants explored the challenges and unmet needs of each of the patient groups, as well as discussed potential solutions. This exercise resulted in the ERIN Manifesto - a document that is meant to inform the work of the ERIN Committee.

Read ERIN Manifesto here: **www.ehc.eu/european-rare-and-inhibitor-network/erin-manifesto/**

ERIN Manifesto

We are people affected by haemophilia A with inhibitors, haemophilia B with inhibitors and extremely rare bleeding disorders, such as clotting factor V, VII and X deficiency, who gathered at European Rare and Inhibitor Network (ERIN) Summit in Ireland from 1-4 December 2022. During the Summit we had an opportunity to come together in numbers that we cannot meet in our local context due to the rarity of our condition. We learned from multidisciplinary care experts, and most importantly – from one another. Together, we outlined needs that we have as a community affected by haemophilia with inhibitors and extremely rare bleeding disorders, the challenges we face in having those needs met and support that we require to have those needs met on European and national levels.

Extremely Rare Bleeding Disorders

We are overlooked as patients and neglected by healthcare professionals, healthcare systems and government. Due to our bleeding disorders, we face challenges in our personal and professional lives that urgently need to be addressed.

Our most urgent unmet needs are access to better treatment, accurate diagnosis, and well-informed, dedicated doctors that focus specifically on extremely rare bleeding disorders. We need to enhance the solidarity for people affected by extremely rare bleeding disorders within and outside the bleeding disorders community.

To be able to achieve this we need to be empowered and independent, feel included, gain knowledge and have access to appropriate information. We need the support from our direct social network – family, friends and colleagues as well as our National Member Organisations and the EHC, as well politicians and industry and other relevant stakeholders.

Haemophilia B with Inhibitors

We are people and families with haemophilia B and inhibitors who face the expected and more importantly – the unexpected every single day. We live with the pain and damage that has been done to our bodies due to the lack of adequate treatment, we live with missed education and job opportunities.

Our most urgent unmet need is access to new prophylactic treatment, as well as adequate multidisciplinary care, especially tailored physiotherapy. We need systematically more time with our doctors, as well as more doctors that focus specifically on rare bleeding disorders. We need more awareness and education about bleeding disorders outside our community – in kindergartens, schools, universities.

To be able to achieve this we need support from our direct social network – family and friends, we need our children to be our guides. We need nurses and doctors to treat with the best options available and to be educated on the latest scientific developments in bleeding disorders treatment, as well as to work in close cooperation among all healthcare specialists – haematologists, physiotherapists, dentists, surgeons, psychosocial experts, nurses etc.

Haemophilia A with Inhibitors

We are a strong community of people affected by haemophilia A with inhibitors that have needed considerable resilience in our path. We have been very isolated and underserved for a long time and lived with limitations in our wishes and desires. Even though significant progress has been made in terms of the treatment that we have, there are many challenges that remain and work needs to be done to maintain the progress and the improved quality of life that has been recently achieved.

We need to ensure that everyone affected by haemophilia A and inhibitors is living a pain-free life and that there is accessible personalised care for everyone, as well as holistic view on haemophilia treatment and access to multidisciplinary care, regardless of where you live. We need a tool to better assess the effects of treatments and interventions.

To be able to achieve this we need a mindset of solidarity and thinking beyond oneself, as well as the ability to transmit the knowledge gained to our local communities. We also need health care providers that are dedicated to acquiring all available knowledge and sharing it with the patients as equal partners. We need standardised policies on bleeding disorders around Europe, as well as high level of advocacy and collaboration between the stakeholders.

We ask that the EHC, our National Member Organisations and the ERIN Committee who will start work on the European Rare and Inhibitor Network in 2023 take this document into account when planning their work.

We ask that in order to improve the treatment and care for all of us, multi-centre research is carried out and new approaches to data collection and treatment development are found.

We ourselves commit to being actively involved in the process of meeting our needs and the needs of those affected by the rare bleeding disorders in our countries, especially those who find themselves outside the bleeding disorders community and the future generations to follow, so that no one is left behind.

Lynn Doyle
Alexander
Christina Burgess
D. Prašćević
Þórey Birta Alexanderdóttir
SANDRA-Marie H.
GABOR EDUARD-Gabor
K. PRAŠĆEVIĆ

COMMUNITY EMPOWERMENT

To empower NMOs and support
their future development

Community Advisory Boards

The EHC offers Community Advisory Boards (CABs) on a need-to-have basis to streamline and centralise external patient advisory 'services' for industry partners seeking European patient input in a fast-changing haemophilia and bleeding disorders landscape, and thus reducing patient advocates' industry conflicts. CABs are individually designed based on both patient community and industry learning and discussion needs. All CABs follow EHC governance, decision-making, participation, confidentiality, minute-taking, evaluation, and final reporting rules set out for this general area of EHC work. The EHC held its first Community Advisory Board (CAB) in 2022 with Sanofi Genzyme.

NMO Support & Community

To offer support and advice to its National Member Organisations (NMOs) for their national patient advocacy efforts and organisational questions and to facilitate the exchange and build stronger connections, each of the NMOs is assigned a liaison from the Steering Committee and EHC staff. Through these liaison tandems regular contact and advocacy support is provided to the NMOs.

Community calls

In 2022, the EHC extended further support to the National Member Organisations (NMOs) by attending relevant NMO events and providing the relevant thematic content when needed and building more meaningful connections with the communities locally.

3 March 2022 - Input of European Rare and Inhibitor Network to Romania Hemophilia Forum

Emergency Displacement Fund

In 2022, the war in Ukraine put an additional strain on the bleeding disorders community, in particular on the NMOs that were dealing with the direct displacement of people with a bleeding disorder and their families. To support these NMOs in their outstanding efforts, the EHC put in place a short-term funding pool, Emergency Displacement Fund. We are immensely grateful to all the NMOs for standing together in these difficult times.

In addition, the EHC was working hard to offer its support to the community in this disturbing and difficult time. Daily, and later weekly updates were sent out in English, Russian and later translated into Ukrainian, offering information regarding leaving the country and the know-how for the arrival in specific countries. The EHC served as a connecting point between the displaced patients and the NMOs welcoming them.

A community call was held on 29 March 2022 to give an update to the entire bleeding disorders community.

Activity Grants and Physical Activity Grants

To support the projects of the NMOs that need an extra nudge, the EHC has put in place a pool of funding that offers project-based funding to the NMOs for their activities designed to improve the quality of life of people living with a rare bleeding disorder in their country.

In 2022, EHC supported following projects through its Activity Grants and Physical Activity Grants:

- Haemophilia sports festival, organised by the French NMO
- World Haemophilia Day Celebration Event and Awareness Campaign by the Maltese NMO
- 9th Annual Parents Meeting, organised by the Portuguese NMO
- Musculoskeletal Programme run by the Romanian NMO
- Renovation of the NMO Website of the Finnish NMO
- An event for women and mothers, organised by the Austrian NMO

EHC Organisational Health Programme

SUSTAINABILITY

To ensure proof of EHC's organisational health andsecure its future development.

In 2022, the EHC took a deeper dive into maintaining good organisational health, by launching a programme that supports the EHC and its National Member Organisations (NMOs) in their pursuit of access to treatment, care and improvements to quality of life for all bleeding disorder patients in their relevant contexts, and helps them to remain healthy and well-functioning organisations.

The EHC-NMO Organisational Health Programme (OHP) was designed to help our NMOs remain strong, help find ways to work with organisational challenges arising due to the pandemic and beyond, and offer mutual support within the EHC network.

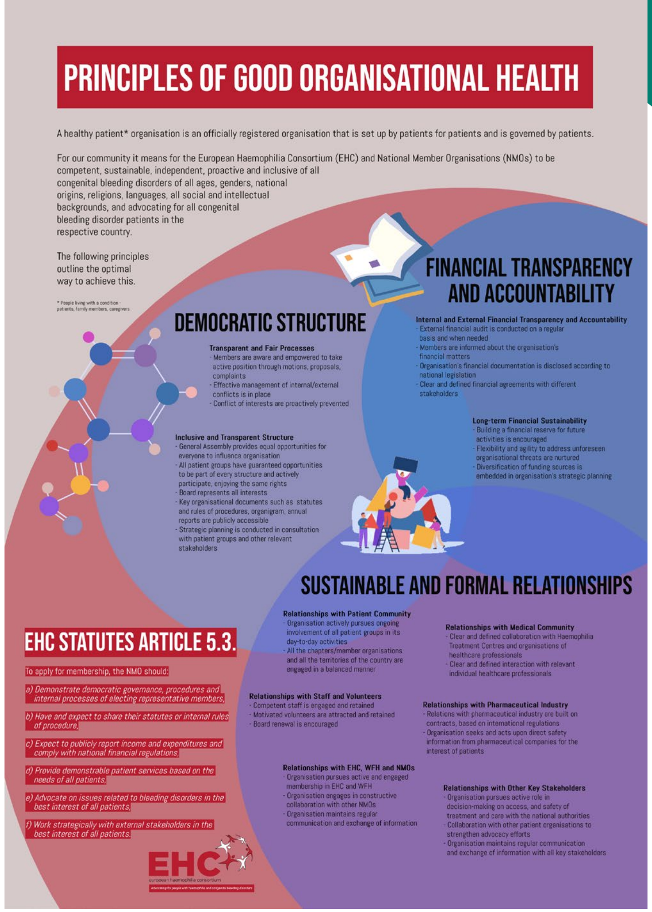
One of the most important aspects of this programme has been that it was co-designed by the whole EHC network, through a team of dedicated volunteers and EHC staff. The co-creation process took the following course:

- **October 2021** - first foundation laid at EHC General Assembly
- **February 2022** - community call introducing programme to NMOs
- **April 2022** - 1st co-creation workshop
- **June 2022** - consultation with the NMOs
- **July 2022** - 2nd co-creation workshop
- **August 2022** - development and review of written report on the proposed programme design by the working group and facilitator
- **September 2022** - mailing of the report to all NMOs as part of General Assembly mailing
- **October 2022** - approval of the OHP by the EHC General Assembly
- **November 2022** - community call to discuss the further steps and plan together with the NMOs

The Organisational Health Programme Working Group played an instrumental role in the development of the programme and consisted of the following volunteers representing a variety of the EHC National Member Organisations:

- **Lorenzo Ghirardi** | Italian NMO
- **Nicolas Giraud** | French NMO
- **Jordan Nedevski** | Bulgarian NMO
- **Brian O'Mahony** | Irish NMO
- **Marius Tănase** | Romanian NMO
- **Mikael Valter** | Swedish NMO
- **Baiba Ziemele** | Latvian NMO
- **Jo Eerens** | EHC Steering Committee

The working group was supported by the EHC Community Programmes Officers Fiona Brennan and Kristine Jansone, and the work was facilitated by two external consultants - Lachezar Afrikanov and Alexander Evtimov.



PRINCIPLES OF GOOD ORGANISATIONAL HEALTH

A healthy patient* organisation is an officially registered organisation that is set up by patients for patients and is governed by patients.

For our community it means for the European Haemophilia Consortium (EHC) and National Member Organisations (NMOs) to be competent, sustainable, independent, proactive and inclusive of all congenital bleeding disorders of all ages, genders, national origins, religions, languages, all social and intellectual backgrounds, and advocating for all congenital bleeding disorder patients in the respective country.

The following principles outline the optimal way to achieve this.

* People living with a condition - patients, family members, caregivers

DEMOCRATIC STRUCTURE

Transparent and Fair Processes
- Members are aware and empowered to take active position through motions, proposals, complaints
- Effective management of internal/external conflicts is in place
- Conflict of interests are proactively prevented

Inclusive and Transparent Structure
- General Assembly provides equal opportunities for everyone to influence organisation
- All patient groups have guaranteed opportunities to be part of every structure and actively participate, enjoying the same rights
- Board represents all interests
- Key organisational documents such as statutes and rules of procedures, organigram, annual reports are publicly accessible
- Strategic planning is conducted in consultation with patient groups and other relevant stakeholders

FINANCIAL TRANSPARENCY AND ACCOUNTABILITY

Internal and External Financial Transparency and Accountability
- External financial audit is conducted on a regular basis and when needed
- Members are informed about the organisation's financial matters
- Organisation's financial documentation is disclosed according to national legislation
- Clear and defined financial agreements with different stakeholders

Long-term Financial Sustainability
- Building a financial reserve for future activities is encouraged
- Flexibility and agility to address unforeseen organisational threats are nurtured
- Diversification of funding sources is embedded in organisation's strategic planning

SUSTAINABLE AND FORMAL RELATIONSHIPS

Relationships with Patient Community
- Organisation actively pursues ongoing involvement of all patient groups in its day-to-day activities
- All the chapters/member organisations and all the territories of the country are engaged in a balanced manner

Relationships with Medical Community
- Clear and defined collaboration with Haemophilia Treatment Centres and organisations of healthcare professionals
- Clear and defined interaction with relevant individual healthcare professionals

Relationships with Staff and Volunteers
- Competent staff is engaged and retained
- Motivated volunteers are attracted and retained
- Board renewal is encouraged

Relationships with Pharmaceutical Industry
- Relations with pharmaceutical industry are built on contracts, based on international regulations
- Organisation seeks and acts upon direct safety information from pharmaceutical companies for the interest of patients

Relationships with EHC, WFH and NMOs
- Organisation pursues active and engaged membership in EHC and WFH
- Organisation engages in constructive collaboration with other NMOs
- Organisation maintains regular communication and exchange of information

Relationships with Other Key Stakeholders
- Organisation pursues active role in decision-making on access, and safety of treatment and care with the national authorities
- Collaboration with other patient organisations to strengthen advocacy efforts
- Organisation maintains regular communication and exchange of information with all key stakeholders

EHC STATUTES ARTICLE 5.3.

To apply for membership, the NMO should:

a) Demonstrate democratic governance, procedures and internal processes of electing representative members,

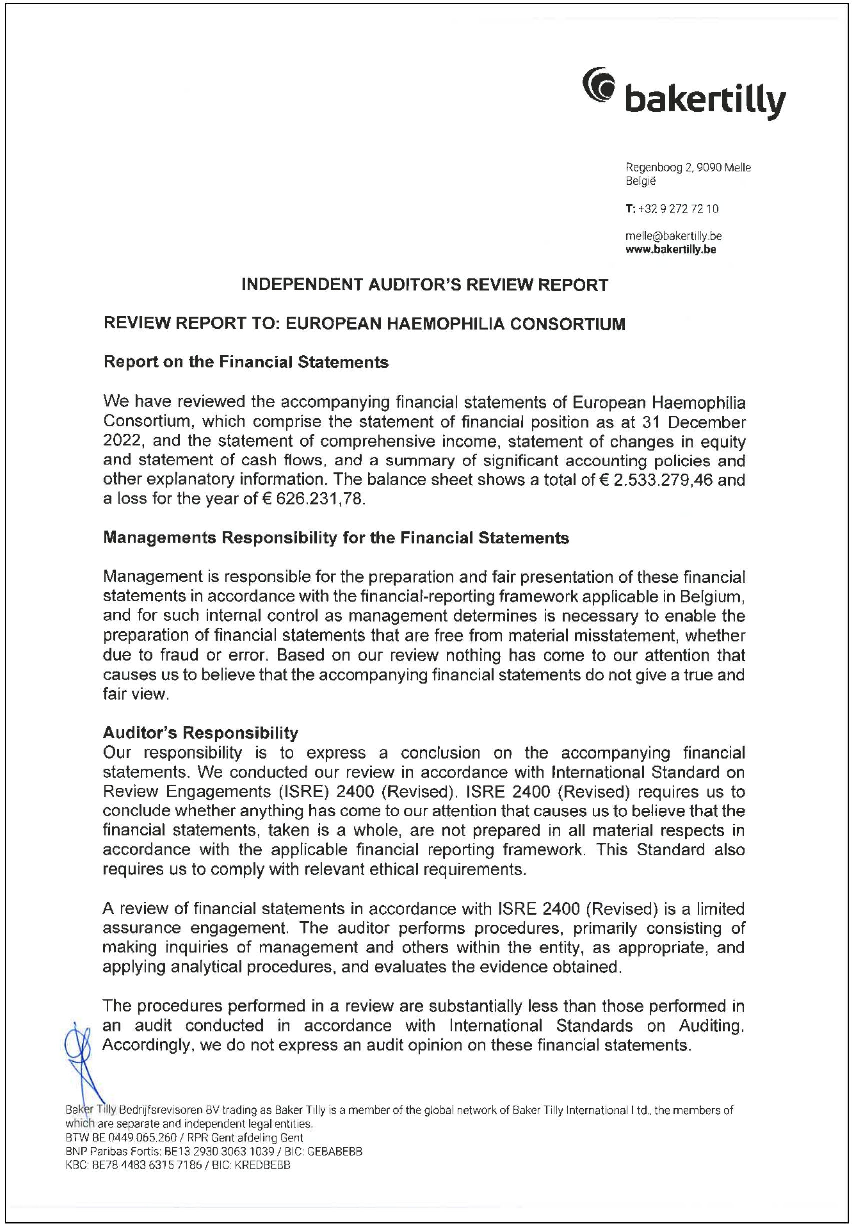
b) Have and expect to share their statutes or internal rules of procedure,

c) Expect to publicly report income and expenditures and comply with national financial regulations,

d) Provide demonstrable patient services based on the needs of all patients,

e) Advocate on issues related to bleeding disorders in the best interest of all patients,

f) Work strategically with external stakeholders in the best interest of all patients.



bakertilly

Regenboog 2, 9090 Melle
België

T: +32 9 272 72 10

melle@bakertilly.be
www.bakertilly.be

INDEPENDENT AUDITOR'S REVIEW REPORT

REVIEW REPORT TO: EUROPEAN HAEMOPHILIA CONSORTIUM

Report on the Financial Statements

We have reviewed the accompanying financial statements of European Haemophilia Consortium, which comprise the statement of financial position as at 31 December 2022, and the statement of comprehensive income, statement of changes in equity and statement of cash flows, and a summary of significant accounting policies and other explanatory information. The balance sheet shows a total of € 2.533.279,46 and a loss for the year of € 626.231,78.

Managements Responsibility for the Financial Statements

Management is responsible for the preparation and fair presentation of these financial statements in accordance with the financial-reporting framework applicable in Belgium, and for such internal control as management determines is necessary to enable the preparation of financial statements that are free from material misstatement, whether due to fraud or error. Based on our review nothing has come to our attention that causes us to believe that the accompanying financial statements do not give a true and fair view.

Auditor's Responsibility
Our responsibility is to express a conclusion on the accompanying financial statements. We conducted our review in accordance with International Standard on Review Engagements (ISRE) 2400 (Revised). ISRE 2400 (Revised) requires us to conclude whether anything has come to our attention that causes us to believe that the financial statements, taken is a whole, are not prepared in all material respects in accordance with the applicable financial reporting framework. This Standard also requires us to comply with relevant ethical requirements.

A review of financial statements in accordance with ISRE 2400 (Revised) is a limited assurance engagement. The auditor performs procedures, primarily consisting of making inquiries of management and others within the entity, as appropriate, and applying analytical procedures, and evaluates the evidence obtained.

The procedures performed in a review are substantially less than those performed in an audit conducted in accordance with International Standards on Auditing. Accordingly, we do not express an audit opinion on these financial statements.

Baker Tilly Bedrijfsrevisoren BV trading as Baker Tilly is a member of the global network of Baker Tilly International Ltd., the members of which are separate and independent legal entities.
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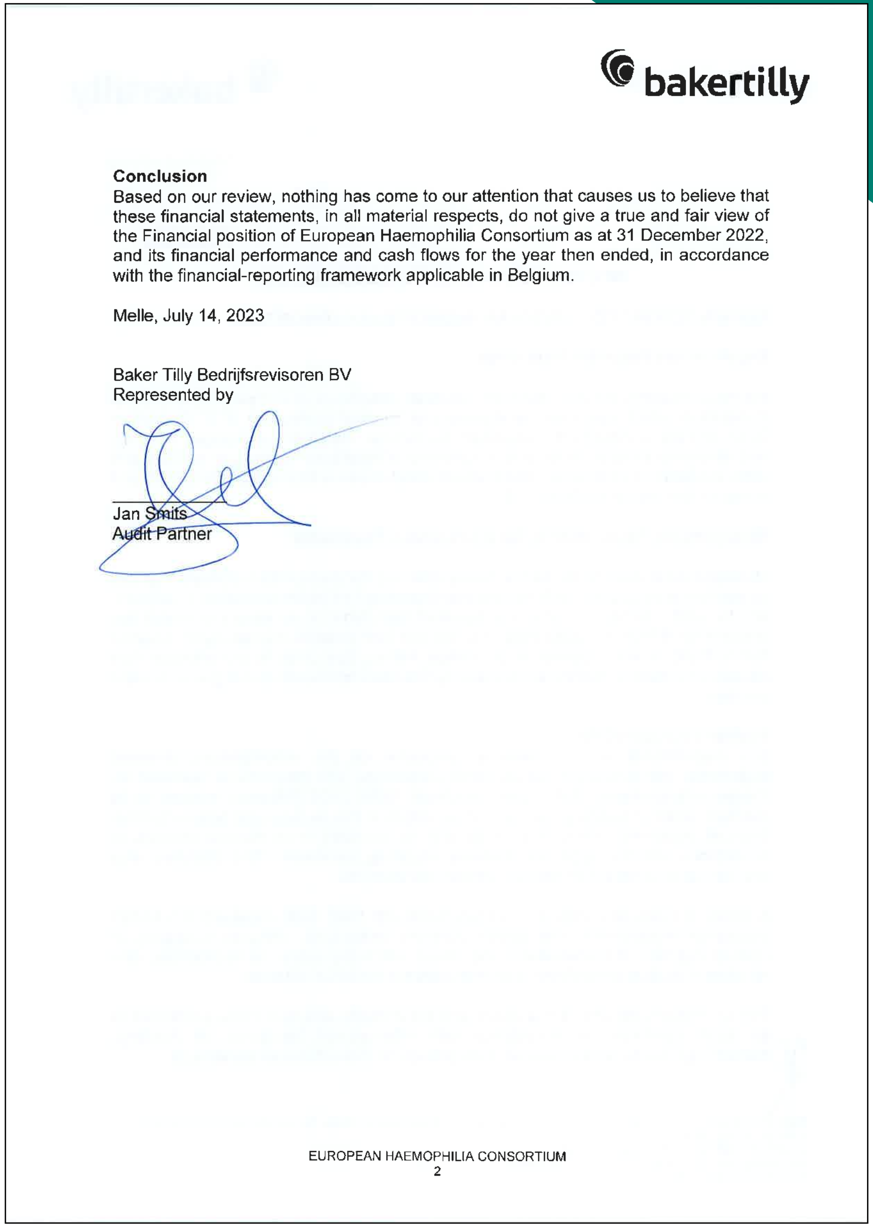


bakertilly

Conclusion
Based on our review, nothing has come to our attention that causes us to believe that these financial statements, in all material respects, do not give a true and fair view of the Financial position of European Haemophilia Consortium as at 31 December 2022, and its financial performance and cash flows for the year then ended, in accordance with the financial-reporting framework applicable in Belgium.

Melle, July 14, 2023

Baker Tilly Bedrijfsrevisoren BV
Represented by

Jan Smits
Audit Partner

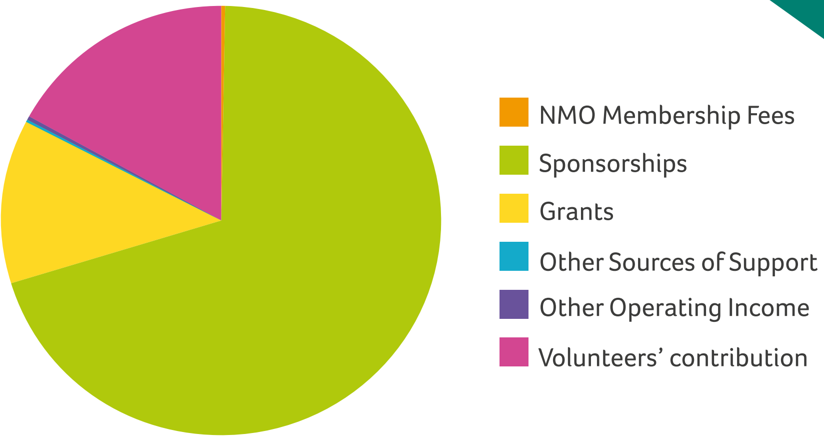
EUROPEAN HAEMOPHILIA CONSORTIUM
2

SUSTAINABILITY

To ensure proof of EHC’s organisational health andsecure its future development.

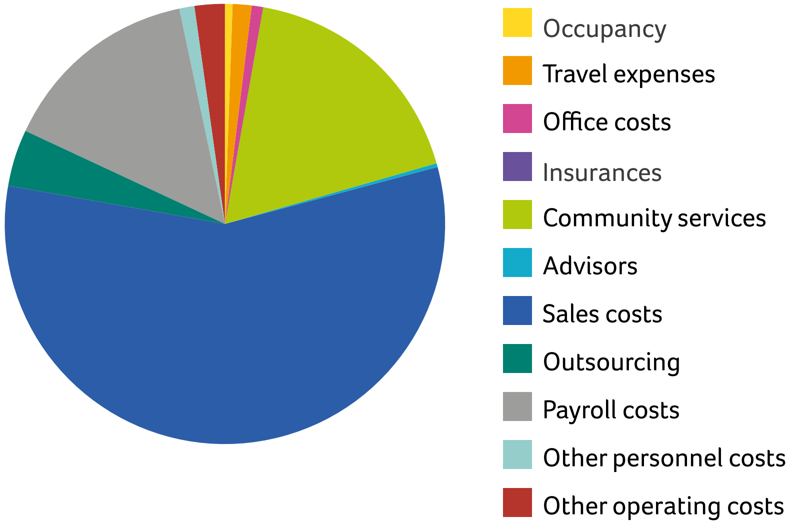
INCOME

INCOME	FINALS (€)
NMO Membership Fees	11250
Sponsorships	1505750.88
Grants	270000
Other Sources of Support	2155
Other Operating Income	4174.84
TOTAL INCOME	**1793330.72**
Volunteers’ contribution	359190



OPERATING COSTS

EXPENDITURE	FINALS (€)
Occupancy	16452.31
Travel expenses	30720.63
Office costs	24465.98
Insurances	528.57
Community services	422044.38
Advisors	9148.38
Sales costs	1353163.01
Outsourcing	104161.67
Payroll costs	346501.58
Other personnel costs	26540.23
Other operating costs	51147.03
TOTAL EXPENDITURE	**2384873.77**



BALANCE SHEET 2022
AS AT 31 DECEMBER 2022

ASSETS	**Debit**	**%**
Buildings and land	474486.84	18.7%
Furniture, vehicles and equipment	12183.57	0.5%
TOTAL FIXED ASSETS	**486670.41**	**19.2%**
Amounts receivable within one year		
- Trade receivables	250970	9.9%
- Other receivables	23767.28	0.9%
Current Investments		
- Other investments	176945.02	7.0%
Cash and cash equivalents	1568739.66	61.9%
Transitory Assests	26187.09	1.0%
TOTAL CURRENT ASSETS	**2046609.05**	**80.8%**
TOTAL ASSETS	**2533279.46**	**100.0%**

LIABILITIES	**Credit**	**%**
Equity	**2206962.92**	**87.1%**
Amounts payable within one year		
- Trade payables	210188.11	8.3%
- Taxes, remuneration and social security	55843.46	2.2%
TOTAL DEBTS	**266031.57**	**10.5%**
Transitory Liabilities	**60284.97**	2.4%
TOTAL LIABILITIES	**2533279.46**	**100.0%**

EHC SUPPORTERS
EHC 2022 CORPORATE GIVING PARTNERS

The EHC wishes to acknowledge and thank its 2022 corporate giving partners for making its work possible.

PLATINUM SPONSORS:

BioMarin | CSL Behring | Pfizer | Roche | Sanofi | Sobi | Takeda

GOLD SPONSORS:

Novo Nordisk | Spark

SILVER SPONSORS:

Bayer | Biotest | Freeline | LFB

The EHC also wishes to thank the following companies for supporting individual 2022 activities and projects:

Sanofi and Sobi kindly sponsored the Youth Leadership Workshop, furthermore Sobi extended their support to the community activities by sponsoring also the Youth Fellowship Programme and Community Advisory Boards (CABs).

Virtual World Haemophilia Week was made possible thanks to the support of Sanofi and Sobi.

The Leadership Conference was kindly sponsored by BioMarin, Sanofi and Sobi.

The VWD Platform received support from Takeda.

Support from Freeline, Roche, Takeda, LFB, BioMarin and Spark helped to keep the EHCucate educational app project up and running, while LFB further supported EHC work with media.

Sobi, Sanofi and Pfizer kindly sponsored the EHC Economics Workshop and the Workshop on New Technologies in Haemophilia Care.

The European Rare and Inhibitor Network, including the ERIN Summit, couldn’t have been carried out without the support of Roche and Sanofi.

The Think Tank activities were made possible by hands-off grants from Sobi, Novo Nordisk, Roche, Takeda, BioMarin, CSL Behring and Sanofi.

Finally, the EHC 2022 Conference received support from Sobi, CSL Behring, Roche, Takeda, Sanofi, BioMarin, and Pfizer as Platinum sponsors; Novo Nordisk and Spark as Gold Sponsors; and Freeline, Bayer, LFB, and Biotest as Silver Sponsors.

VOLUNTEERS

In 2022, the work of the EHC was supported by a total of 179 volunteers, who all together dedicated 5986,5 hours of their time to the EHC. The EHC is immensely grateful to everybody listed below for their energy and dedication! Thank you!

Rezan Abdul-Kadir, Aizat Aidarbekova, Jacob Andersen, Enea Atroce, Cijan Ay, Fariba Baghaei, Paul Batty, Marion Bräuer, Nathalie Bere, Jan Blatný, Lara Bloom, Ana Boban, Simone Boselli, Annette Bowyer, Ellie Brennan, Christina Burgess, Jessica Byrne, Shannon Carey, Laura Canning, Chanel Cassar, Cristina Catarino, Dominik Cepic, Panagiotis Christoforou, Yannick Collé, Massimo Colombo, Michel Coppens, Miguel Crato, Gregor Cuzak, Roseline D'Oiron, Piet de Kleijn, Manon Degenaar-Dujardin, Milica Dimic, Karola Diósi, Alison Dougall, Nathan Doyle, Mariëtte Driessens, Marija Aloa Vera Drzmanoska, Vincent Dumez, Jo Eerens, Yasemin Erbas, Carmen Escuriola-Ettingshausen, Albert Farruggia, Karin Fijnvandraat, Laurent Frenzel, Eva Funding, Giulia Gabrielli, Roberta Galtieri, Anya Ganeva, Isabella Garagiola, Lorenzo Ghirardi, Paul Giangrande, Nicolas Giraud, Wim Goetsch, Keith Gomez, Jenny Goudemand, Matej Grah, Willie Greevenbroek, Jack Grehan, Evelyn Grimberg, Dan Hart, Victoria Hedley, Cedric Hermans, Magnus Heunicke, Anneliese Hilger, Nicolaj Holm Ravn Faber, Anke-Peggy Holtorf, Eke Hullegie, Sara Hutton, Lone Hvitfeldt Poulsen, Vince Jenkins, Bernd Jilma, Matt Johnson, Mirko Jokic, Gavin Jones, Radek Kaczmarek, Christine Keipert, Kate Khair, Marianna Kladi, Robert Klamroth, Barbara Konkle, Ilmar Kruis, Vincenzo La Mura, Elise Lammertyn, Frank Leebeek, Diana Lighezan, David Lillicrap, Johnny Mahlangu, Sunjeev Maini, Alexandre Majet, Mike Makris, Mihaela Iona Malita, Maria Elisa Mancuso, Tatjana Markovic, Evelien Mauser-Bunschoten, Giuseppe Mazza, Donal Mc Cann, William McKeown, Geoff McDonough, Paul McLaughlin, Maia Meier, Stephan Meijer, Flavio Minelli, Connie Montgomery, Greta Mulder, Lars Münter, Jordan Nedevski, Beatrice Nolan, Declan Noone, Cristina Novembrino, Jamie O'Hara, Jim O'Leary, Brian O'Mahony, Johannes Oldenburg, David Page, Roberta Palla, Ana Pastor, Katja Peltoniemi, Teresa Pereira, Martine Pergent, Flora Peyvandi, Glenn Pierce, Kelly Plueschke, Debra Pollard, Sheila Radhakrishnan, Stefan Radovanovic, Priyanka Raheja, Savita Rangarajan, Julia Rauscher, Francesco Rodeghiero, Olivia RomeroLux, Frits Rosendaal, Thomas Sannié, Anja Schiel, Uwe Schlenkrich, Marie Louise Schougaard, Roger Schutgens, Martin Sedmina, Omid Seidizadeh, David Silva, Glenda Silvester, Suthesh Sivalaparatnam, Mark Skinner, Egidijus Sliauzys, Cees Smit, Luigi Solimeno, Sophie Susen, Marius Tanase, Anna Rita Tavares, Renske ten Ham, Merel Timmer, Jelisaveta Todorovic, Ana Toledo Chavarri, Anna Tollwé, Megan Townsend, Joanne Traunter, Nanda Uitslager, Sheela Upadhyaya, Minette van der Ven, Tessa van Gastel, Waander van Heerde, Mirna Van Steenbergen, Swen Van Steenbergen, Timon Van Steenbergen, Thierry Vanden Driessche, Kristof Vanfraechem, Mikael Vater, Allison Wheeler, Durhane Wong Reider, Laurence Woollard, Yuri Zhulyov, Baiba Ziemele.

SERVICE PROVIDERS

The work of the EHC would not be possible without the support of all of its service providers including:

- Advantage Point
- Alina Oleksiienko
- Art of e-Learning
- Assembly Voting
- Barretstown
- Breex
- Brenda Cecilia Padilla Rodriguez
- Canopée Studio
- CitrusSuite
- Conference Organisers
- Copenhagen Economics
- David Mendes da Silva
- EASY Noise Control
- Elliott Laub
- Fiona Robinson
- Georghia Michael
- Goran Kapetanovic
- Haemnet
- Han Han Li
- Ia Brix Ohmann
- Jeff Stanford
- Jens Steen Larsen from Larsen & Co
- Joseph Carr
- Julia Krivkina
- Lachezar Afrikanov
- Mak Omanovic
- Mark Brooker
- Matt Evans
- Netika
- Olga Miniuk
- OPEN Health
- Sherpah Video Projects
- Svetoslava Stoyanova
- UniWeb
- Voilà
- Volodymyr Zhyvytskyi
- Weichie
- Yaska

Thank you for supporting the European bleeding disorders community!

The EHC is also particularly grateful for the fully pro-bono services and support provided by Covington & Burling LLP.

THANK YOU!

Driven by Our Patients

CSL Behring is proud to work together with the European Haemophilia Consortium to help ensure that patients' needs are met.

CSL Behring

PFIZER HAS DRIVEN INNOVATION IN HAEMOPHILIA FOR OVER 40 YEARS.[1,2]

Through our partnership with the European Haemophilia Consortium (EHC) we continue to:

- ✓ develop educational patient resources & programs
- ✓ amplify patient voices
- ✓ support the haemophilia community

Pfizer has supported the following EHC-organised activities*:

EHC conferences

Think Tank

Leadership conferences

Health Economics Workshop

Pfizer is committed to reshaping hemophilia care

Discover what may be on the horizon & learn why gene therapy is being researched for the treatment of haemophilia: https://www.haemhorizon.eu/

REFERENCES:

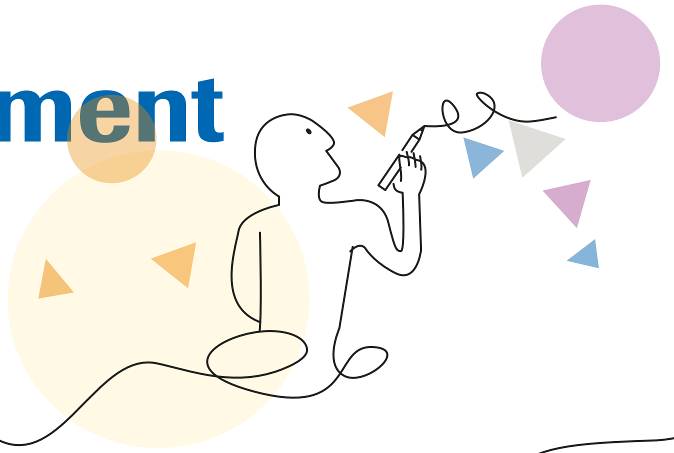
1. *Toole JJ et al. Molecular cloning of a cDNA encoding human antihaemophilic factor. Nature. 1984; 312(5992):342-7.*
2. *Pfizer. Hemophilia. https://www.pfizer.com/disease-and-conditions/hemophilia. (Accessed Aug 23, 2023)*

**These activities are independently initiated, organised, and developed by the EHC.*

PP-UNP-GLB-1381
August 2023

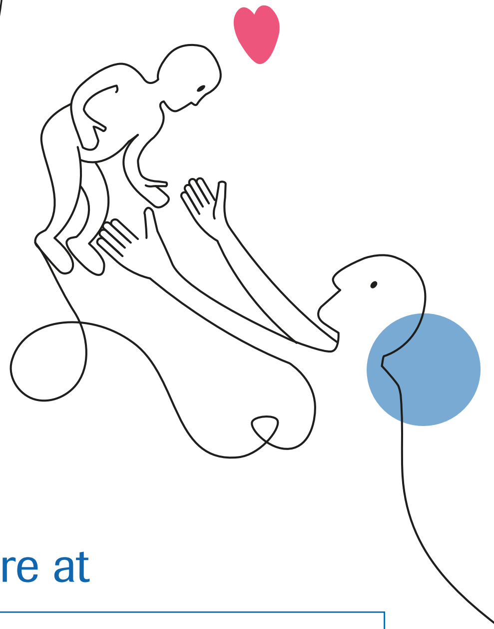
Prophylaxis Treatment

for those who need it most



Roche is committed to supporting
rapid, broad and sustainable access
to treatments…

… for
all people with haemophilia A
around the world…



… to help **you live the life you want**

Find out more at

www.roche.com/haemophilia

July 2023, Veeva document M-XX-00014138

We are an innovative global healthcare company, driven by one purpose: we chase the miracles of science to improve people's lives.

We are dedicated to transforming the practice of medicine by working to turn the impossible into the possible. We provide potentially life-changing treatment options and life-saving vaccine protection to millions of people globally, while putting sustainability and social responsibility at the center of our ambitions.

sanofi

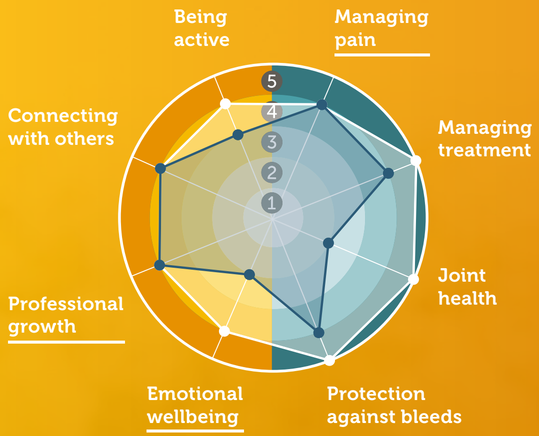
LIBERATE
LIFE

Living life beyond **haemophilia**

Explore what liberation means for you

At Sobi, we strive to empower every person with haemophilia to live the life they want.

Liberation Map is a tool created together with the haemophilia community that may help people with haemophilia to pursue what matters most to them. **Liberation Map** helps people identify possible health and life goals, share them with their care team and discuss how to achieve them.



You can access the **Liberation Map** in the below countries by clicking on the relevant flag:

sobi
rare **strength**

Swedish Orphan Biovitrum AB (publ) SE-112 76 Stockholm, Sweden +46(0)18 697 20 00
Date of preparation: April 2022. NP-21487

Scan here for further information about the Liberation Map

Better Health, Brighter Future

At Takeda we believe every bleed matters, and that all men and women with a rare bleeding disorder, wherever they live, should have access to comprehensive, personalized care. We are committed to raising the voices of people living with rare bleeding disorders to ensure their meaningful involvement in decision making.

EHC Think Tank

Patient education app EHCucate

Symposium during EHC 2022

VWD platform

Takeda is proud to support the European Haemophilia Consortium (EHC) and is committed to raising expectations for the future for persons living with rare bleeding disorders by championing early diagnoses, effective bleed protection and personalized care. We look forward to continuing to support the work of the EHC throughout 2023.

VWD, von Willebrand disease.

July 2023 | C-ANPROM/INT/CORP/0067

Takeda

Why do only men get haemophilia?

Newsflash... they don't! In fact, women can have haemophilia too.

More can and should be done to address the needs of women and girls with bleeding disorders (WGBD). Improving their timely diagnosis is critical and a necessary first step in this endeavour. It is particularly important that heavy menstrual bleeding (HMB) – the most common bleeding symptom of bleeding disorders in women and girls – is given the attention it warrants.[1,2,3,4,5,6,7]

Visit **www.changinghaemophilia.com** to read how Novo Nordisk is raising awareness of heavy menstrual bleeding.

1. James AH. More than menorrhagia: a review of the obstetric and gynaecological manifestations of bleeding disorders. Haemophilia. 2005;11(4):295-307.doi:10.1111/j.1365-2516.2005.01108.x **2.** Halimeh S. Menorrhagia and bleeding disorders in adolescent females. Hamostaseologie. 2012;32(1):45-50. doi:10.5482/ha-1181 **3.** Noone D, Skouw-Rasmussen N, Lavin M, van Galen KPM, Kadir RA. Barriers and challenges faced by women with congenital bleeding disorders in Europe: Results of a patient survey conducted by the European Haemophilia Consortium. Haemophilia. 2019;25(3):468-474. doi:10.1111/hae.13722 **4.** Byams VR, Kouides PA, Kulkarni R, et al. Surveillance of female patients with inherited bleeding disorders in United States Haemophilia Treatment Centres. Haemophilia. 2011;17 Suppl 1(0 1):6-13. doi:10.1111/j.1365-2516.2011.02558.x **5.** Kadir RA, Economides DL, Sabin CA, Pollard D, Lee CA. Assessment of menstrual blood loss and gynaecological problems in patients with inherited bleeding disorders. Haemophilia. 1999;5(1):40-48. doi:10.1046/j.1365-2516.1999.00285.x **6.** Lavin M, Aguila S, Dalton N, et al. Significant gynecological bleeding in women with low von Willebrand factor levels. Blood Adv. 2018;2(14):1784-1791.doi:10.1182/bloodadvances.2018017418 **7.** Atiq F, Saes JL, Punt MC, et al. Major differences in clinical presentation, diagnosis and management of men and women with autosomal inherited bleeding disorders. EClinicalMedicine. 2021;32:100726. Published 2021 Jan 29.doi:10.1016/j.eclinm.2021.100726

Date of preparation August 2023. HQ23CH00010

changing haemophilia®

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novo nordisk®

EHC
european haemophilia consortium
Advocating for people with haemophilia and congenital bleeding disorders

European Haemophilia Consortium aisbl
Rue de la Loi 28, 1040 Brussels, Belgium
e: office@ehc.eu **w:** ehc.eu
EHC_Haemophilia European Haemophilia Consortium ehc_haemophilia

European Haemophilia Consortium